

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549**

**FORM S-1  
REGISTRATION STATEMENT**

**UNDER  
THE SECURITIES ACT OF 1933**

**Neurogene Inc.**

(Exact name of registrant as specified in its charter)

Delaware  
(State or other jurisdiction of  
incorporation or organization)

2834  
(Primary Standard Industrial  
Classification Code Number)

98-0542593  
(I.R.S. Employer  
Identification No.)

535 W 24<sup>th</sup> St.  
5th Floor  
New York, NY 10011  
(855) 508-3568

(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

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**Approximate date of commencement of proposed sale to the public:** From time to time after the effective date of this Registration Statement.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box.   
If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act.

**The Registrant hereby amends this registration statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment that specifically states that this registration statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933, as amended (the "Securities Act"), or until this registration statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.**

**The information in this prospectus is not complete and may be changed. The selling stockholders named in this prospectus may not sell these securities until the registration statement filed with the Securities and Exchange Commission (the “SEC”) is effective. This prospectus is not an offer to sell these securities, and it is not soliciting an offer to buy these securities, in any jurisdiction where the offer or sale is not permitted.**

**SUBJECT TO COMPLETION, DATED DECEMBER 2, 2024**

**PRELIMINARY PROSPECTUS**



**Neurogene Inc.**  
**4,000,042 Shares**  
**Common Stock**  
**Offered by the Selling Stockholders**

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This prospectus relates to the proposed resale or other disposition by the selling stockholders identified herein (the “Selling Stockholders”) of up to (i) 1,835,000 shares of our common stock, par value \$0.000001 per share (“Common Stock”) and (ii) 2,165,042 shares of Common Stock issuable upon the exercise of pre-funded warrants (the “Pre-Funded Warrants”). The shares of Common Stock registered by this prospectus are referred to herein as the “Resale Shares.”

The Resale Shares were issued and sold to accredited investors in a private placement (the “PIPE”), which closed on November 5, 2024. We are not selling any Resale Shares under this prospectus and will not receive any of the proceeds from the sale or other disposition of Resale Shares by the Selling Stockholders. Upon any exercise of the Pre-Funded Warrants by payment of cash, however, we will receive the nominal cash exercise price paid by the holders of the Pre-Funded Warrants. We intend to use those proceeds, if any, for general corporate purposes.

The Selling Stockholders may sell the Resale Shares on any national securities exchange or quotation service on which the securities may be listed or quoted at the time of sale, on the over-the-counter market, in one or more transactions otherwise than on these exchanges or systems, such as privately negotiated transactions, or using a combination of these methods, and at fixed prices, at prevailing market prices at the time of the sale, at varying prices determined at the time of sale, or at negotiated prices. See the disclosure under the heading “Plan of Distribution” elsewhere in this prospectus for more information about how the Selling Stockholders may sell or otherwise dispose of their Resale Shares hereunder.

The Selling Stockholders may sell any, all or none of the securities offered by this prospectus and we do not know when or in what amount the Selling Stockholders may sell their Resale Shares hereunder following the effective date of the registration statement of which this prospectus forms a part. Discounts, concessions, commissions and similar selling expenses attributable to the sale of the Resale Shares will be borne by the Selling Stockholder. We will pay certain fees and expenses (other than discounts, concessions, commissions and similar selling expenses) incident to the registration of the Resale Shares with the SEC.

You should carefully read this prospectus and any applicable prospectus supplement before you invest in any of the securities being offered.

Our Common Stock is traded on The Nasdaq Global Select Market under the symbol “NGNE.” On December 2, 2024, the last reported sale price for our Common Stock was \$24.57 per share.

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**An investment in our securities involves a high degree of risk. You should carefully consider the information under the heading “[Risk Factors](#)” beginning on page 8 of this prospectus and any applicable prospectus supplement. We are a “smaller reporting company” as defined by Rule 12b-2 of the Exchange Act and are subject to reduced public company reporting requirements. Neither the SEC nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.**

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The date of this prospectus is \_\_\_\_\_, 2024

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## ABOUT THIS PROSPECTUS

This prospectus is part of a registration statement on Form S-1 that we filed with the SEC using a “shelf” registration process. Under this shelf registration process, from time to time, the Selling Stockholders may sell the securities described herein in one or more offerings as described in this prospectus.

We have not authorized anyone to provide you with any information or to make any representation other than the information and representations contained in or incorporated by reference into this prospectus or any applicable prospectus supplement. We and the Selling Stockholders take no responsibility for, and can provide no assurances as to the reliability of, any other information that others may give you. The information contained in this prospectus (and in any supplement or amendment to this prospectus) or any related free writing prospectus, and the documents incorporated by reference herein and therein, are accurate only as of their respective dates, regardless of the time of delivery of this prospectus, any applicable prospectus supplement or any related free writing prospectus, or any sale of a security. We urge you to read carefully this prospectus (as supplemented and amended) before deciding whether to invest in any of the shares of common stock being offered.

This prospectus contains summaries of certain provisions contained in some of the documents described herein, but reference is made to the actual documents for complete information. All of the summaries are qualified in their entirety by the actual documents. Copies of some of the documents referred to herein have been filed, will be filed or will be incorporated by reference as exhibits to the registration statement of which this prospectus is a part, and you may obtain copies of those documents as described below under the section entitled “Where You Can Find More Information.”

In this prospectus, unless the context otherwise requires, the terms “Neurogene,” the “Company,” “we,” “us,” and “our” refer to Neurogene Inc. (formerly Neoleukin Therapeutics, Inc.), a Delaware corporation, and Neurogene Inc., a Nevada corporation, the wholly owned operating subsidiary of the Delaware corporation. The name “Neurogene” is a trademark of the Company in the United States. This report also contains references to registered marks, trademarks and trade names of other companies that are property of their respective holders. All other trademarks, registered marks and trade names appearing in this report are the property of their respective holders.

## CAUTIONARY STATEMENT CONCERNING FORWARD-LOOKING STATEMENTS

This prospectus contains “forward-looking statements” within the meaning of the federal securities laws, which statements are subject to substantial risks and uncertainties and are based on estimates and assumptions. All statements, other than statements of historical facts, including statements concerning our plans, objectives, goals, strategies, future events, future revenues or performance, financing needs, plans or intentions relating to products and markets, and business trends and other information referred to under the sections entitled “Risk Factors,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” and “Business” are forward-looking statements. In some cases, you can identify forward-looking statements by terms such as “anticipate,” “believe,” “can,” “could,” “design,” “develop,” “estimate,” “expect,” “intend,” “objective,” “may,” “might,” “plan,” “potential,” “predict,” “should,” “will,” “would,” or the negative of these terms, and similar expressions intended to identify forward-looking statements. Forward-looking statements are not historical facts, and reflect our current views with respect to future events. Given the significant uncertainties, you should not place undue reliance on these forward-looking statements.

There are a number of risks, uncertainties and other factors that could cause our actual results to differ materially from the forward-looking statements expressed or implied in this prospectus. Such risks, uncertainties and other factors include, among others, the following:

- We have a limited operating history, have not completed any clinical trials, and have no products approved for commercial sale, and our results may vary from quarter to quarter.
- We will require substantial additional capital to finance our operations in the future. If we are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce or eliminate clinical trials, product development programs or future commercialization efforts.
- We have incurred significant losses since inception, expect to incur significant losses for the foreseeable future and may not be able to achieve or sustain profitability in the future. We have no products for sale, have not generated any product revenue and may never generate product revenue or become profitable.
- NGN-401 and our other programs are in early stages of development and may fail in development or suffer delays that materially and adversely affect their commercial viability. If we or our current or future collaborators are unable to complete development of, or commercialize, our product candidates, or experience significant delays in doing so, our business will be materially harmed.
- We are substantially dependent on the success of our most advanced product candidate, NGN-401, and our ongoing and anticipated clinical trials of such candidate may not be successful.
- Delays in developing our manufacturing capabilities or failure to achieve operating efficiencies from such capabilities may require us to devote additional resources and management time to manufacturing operations and may delay our product development timelines.
- We have a number of academic collaborations, and currently rely on our collaboration with the University Court of the University of Edinburgh (the “University of Edinburgh”) for certain aspects of our preclinical research and development programs, including working in collaboration to discover and preclinically develop potential product candidates for our near-term future pipeline. Failure or delay of the University of Edinburgh or any other collaborator to fulfil all or part of its obligations under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of the relationship would materially harm our business.
- In order to successfully implement our plans and strategies, we will need to grow the size of our organization and we may experience difficulties in managing this growth.
- The regulatory approval processes of the U.S. Food and Drug Administration (“FDA”) and other comparable foreign regulatory authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for our product

candidates, we will not be able to commercialize, or will be delayed in commercializing, such product candidates, and our ability to generate revenue will be materially impaired.

- The market price of our common stock may continue to be volatile.
- We may be required to allocate resources to fulfilling the requirements of the Contingent Value Rights Agreement entered into in connection with the Reverse Merger related to certain legacy lease obligations, which may take away from our core programs and create a distraction for our management and employees.
- Future sales of shares by existing stockholders could cause our stock price to decline.
- Our executive officers, directors and principal stockholders have the ability to control or significantly influence all matters submitted to our stockholders for approval.

There may be other factors that may cause our actual results to differ materially from the forward-looking statements expressed or implied in this prospectus, including factors disclosed in “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.” You should evaluate all forward-looking statements made in this prospectus in the context of these risks and uncertainties.

We caution you that the risks, uncertainties, and other factors referred to above and elsewhere in this prospectus may not contain all of the risks, uncertainties and other factors that may affect our future results and operations. Moreover, new risks will emerge from time to time. It is not possible for our management to predict all risks. In addition, we cannot assure you that we will realize the results, benefits or developments that we expect or anticipate or, even if substantially realized, that they will result in the consequences or affect us or our business in the way expected.

Any forward-looking statements contained in this prospectus speak only as of the date hereof and not of any future date, and we expressly disclaim any intent to update any forward-looking statements, whether as a result of new information, future events or otherwise.

## PROSPECTUS SUMMARY

*This summary may not contain all the information that you should consider before investing in securities. You should read the entire prospectus carefully, including the sections titled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” and our consolidated financial statements and the related notes included elsewhere in this prospectus, before making an investment decision.*

### Company Overview

Despite recent scientific advances in genetics, most neurological diseases, particularly those with devastating consequences to patients, are left untreated. Conventional gene therapy is an attractive potential treatment approach for only a limited number of monogenic diseases due to the challenges caused by the complex biology of neurological diseases and by inherent variable transgene uptake and expression. We are a clinical-stage biotechnology company committed to overcoming these limitations and turning today’s complex devastating neurological diseases into treatable conditions. By harnessing our proprietary transgene regulation technology, EXACT™ (Expression Attenuation via Construct Tuning), we are building a robust and differentiated product portfolio of genetic medicines for rare neurological diseases with high unmet need not otherwise addressable by conventional gene therapy. Our EXACT approach leverages key scientific breakthroughs, including gene transfer technology, microRNA-based genetic circuits, and adeno-associated virus (“AAV”) delivery, and is designed to deliver therapeutic levels of transgene to key areas of the brain that underlie neurological disease pathology.

Our first clinical-stage program to utilize the EXACT platform is NGN-401, which is in development for the treatment of Rett syndrome, a disease with a patient population that has a significant unmet need, and that ultimately progresses to substantial neurological and physical impairment and premature death. We are conducting a Phase 1/2 open-label, multi-center clinical trial of NGN-401 gene therapy for the treatment of female patients with classic Rett syndrome that is assessing the safety, tolerability, and efficacy of NGN-401 at a dose of 1E15 vg. In November 2024, we announced the initiation an adult/adolescent cohort that will evaluate NGN-401 in participants ages 16 and above. Clinical grade NGN-401 was manufactured at our manufacturing facility, and is being used for dosing in the Phase 1/2 clinical trial. NGN-401 is delivered using a one-time intracerebroventricular (“ICV”) procedure, which we believe is the most suitable route of administration to achieve optimal biodistribution in key regions of the brain and other parts of the nervous system.

The NGN-401 clinical trial has been initiated in three regions - the United States, the United Kingdom (“UK”) and Australia, after clearance of our Investigational New Drug (“IND”) application by the FDA in January 2023, the clearance of the clinical trial application by the UK Medicines and Healthcare Products Regulatory Agency in January 2024, and acknowledgement from the Australian Therapeutic Goods Administration and approval from the Human Research Ethics Committee in May 2024. Participants have been dosed in all three regions.

On November 11, 2024, we announced positive interim clinical data in all participants in the 1E15 vg cohort (low-dose cohort, n=4) from the Phase 1/2 clinical trial with a data cut-off date of October 17, 2024.. Consistent, concordant and durable improvements were observed as measured from baseline across multiple Rett syndrome clinical assessments. Participants also achieved meaningful gains of skills and developmental milestones in hand function, gross motor, and communication, all of which are core clinical domains of Rett syndrome. These gains of skills and developmental milestones would not be expected to occur when compared and contextualized against the natural history of Rett syndrome. Additionally, objective improvements in autonomic function were recorded.

Consistent improvements were observed in the following:

	CGI-I		CGI-S Total Score		RSBQ		Gain of Skills, Developmental Milestones and Symptom Improvement in RTT Clinical Domains				
	Improved?	How many points?*	Improved?	How many points?	Improved?	How many points? (% Change)	Hand Function	Gross Motor	Communication	Autonomic	Attentiveness
LD:1 15 mos. post-NGN-401	✓	2 pts.			✓	10 pts. (-28%)	✓	✓	✓	✓	✓
LD:2 12 mos. post-NGN-401	✓	2 pts.	✓	1 pt.	✓	32 pts. (-52%)	✓	✓	✓	✓	✓
LD:3 9 mos. post-NGN-401	✓	2 pts.			✓	5 pts. (-29%)	✓	✓		✓	✓
LD:4 3 mos. post-NGN-401	✓	2 pts.			✓	8 pts. (-28%)	✓			✓	✓

As of data cut-off date of October 17, 2024

\*Each participant achieved a 2-point improvement, or “much improved” from baseline

CGI-I = Clinician Global Impression-Improvement; CGI-S = Clinician Global Impression-Severity; RSBQ = Rett Syndrome Behavior Questionnaire

We also announced safety and tolerability data from the Phase 1/2 clinical trial with a data cut-off date of October 17, 2024. NGN-401 has been well-tolerated and shows a favorable safety profile at a dose of 1E15 vg (n=5, low-dose cohort), with no treatment-related serious adverse events (“SAEs”) at that dose. Treatment-related AEs at the 1E15 vg dose have been mild/Grade 1. One Grade 1 AE of abnormal sural (sensory) nerve conduction study at the 1E15 vg dose level was observed, and the participant is asymptomatic. Treatment-related AEs in the first two participants dosed with 3E15 vg of NGN-401 (high-dose cohort) included Grade 2 and Grade 3 elevations in alanine aminotransferase and aspartate aminotransferase levels, and Grade 2 decreased platelets, which resolved with steroids. One Grade 1 AE of abnormal sural (sensory) nerve conduction study at the 3E15 vg dose level was observed, and the participant is asymptomatic. Across both dose levels, there were no signs or symptoms indicating MeCP2 overexpression toxicity, and there were no ICV procedure-related AEs.

On November 11, 2024, we were notified of an SAE in the third participant who was dosed with 3E15 vg (high-dose cohort) on November 5, 2024. The participant subsequently died following complications from a rare and life-threatening hyperinflammatory syndrome associated with systemic exposure to high doses of AAV. Upon notification of the SAE, the Company consulted with the Data Safety Monitoring Board (DSMB) regarding the continued dosing of participants in the 1E15 vg (low-dose cohort). Following this consultation, the DSMB did not express concerns regarding the continued dosing of participants in the low-dose cohort. The Company also paused further use of the 3E15 vg dose (high dose cohort) on November 11, 2024 and does not plan to enroll any further participants at that dose level. The U.S. Food and Drug Administration (“FDA”) completed a review of the safety data for NGN-401 and allowed us to proceed with the Phase 1/2 trial using the 1E15 vg dose for both the pediatric and adolescent/adult cohorts. We are updating the trial protocol to remove the 3E15 vg dose. We will also incorporate the 1E15 vg dose in our future registrational trial design planning.



The baseline demographics of the first five participants who received 1E15 vg NGN-401 (low dose) in Cohort 1 and the first two participants who received 3E15 vg NGN-401 (high dose) in Cohort 2 include:

	Low-Dose Cohort 1 (1E15 vg)					High-Dose Cohort 2 (3E15 vg)	
	Participant 1 (LD:1)	Participant 2 (LD:2)	Participant 3 (LD:3)	Participant 4 (LD:4)	Participant 5 (LD:5)	Participant 1 (HD:1)	Participant 2 (HD:2)
<b>Age at Dosing in Years</b>	7	4	6	7	6	5	7
<b>MECP2 Mutation Severity</b>	Mild	Severe	Severe	Severe	Severe	Severe	Unclassified
<b>Baseline Disease Severity as Indicated by CGI-S Score</b>	4 (moderately ill)	5 (markedly ill)	5 (markedly ill)	5 (markedly ill)	5 (markedly ill)	5 (markedly ill)	4 (moderately ill)
<b>Time Post Treatment with NGN-401 in Months</b>	~15	~12	~9	<6	~1	~5	~2

In November 2024, we also shared that we have gained alignment with the FDA on our CMC scale-up and potency assay for the program. We expect to share information about our registration trial design in the first half of 2025 and to announce additional Phase 1/2 clinical data from the trial in the second half of 2025.

NGN-401 has been selected by the Center for Biologics Evaluation and Research at the FDA to participate in the FDA’s Support for clinical Trials Advancing Rare disease Therapeutics (“START”) Pilot Program based on potential for clinical benefits and clinical development program readiness. As part of the START Program, we will have opportunities for enhanced communications with the FDA, with the aim to further accelerate the pace of NGN-401’s development. In addition, NGN-401 has received Regenerative Medicine Advanced Therapy (“RMAT”) designation from the FDA. RMAT designation is granted for regenerative medicines intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition, and with preliminary clinical evidence that indicates that the drug has the potential to address unmet medical needs. Benefits of the RMAT designation program include all the benefits of Fast Track and Breakthrough Therapy designation programs, including early and frequent communications with FDA senior managers, intensive guidance on efficient drug development and eligibility for an Accelerated Approval pathway and Priority Review.

We believe that our EXACT platform has broad applicability in complex neurological diseases not otherwise easily addressable by conventional gene therapy. In addition to our Rett syndrome program, we have multiple programs in the discovery stage. We anticipate advancing one program into clinical development in 2025.

In addition to NGN-401, we have also been pursuing a conventional gene therapy program in a Phase 1/2 clinical trial of NGN-101 for the treatment of CLN5 Batten disease. This patient population has a significant unmet need, and experiences extensive neurological and physical impairment leading to blindness, loss of motor function and early mortality. Our Phase 1/2 clinical trial of NGN-101 was the first trial to assess the treatment of both neurodegenerative and ocular disease manifestations of Batten disease. In November 2024, we announced that the Company does not expect to move forward with the NGN-101 CLN5 Batten disease gene therapy program; given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. We are currently evaluating options for the program.

We also established a fully operational current good manufacturing practices (“cGMP”) facility in Houston, Texas used to manufacture current and future product for research, toxicology and clinical studies. We believe that our in-house manufacturing capabilities better enable control of product quality and development timelines, strategic pipeline and financial flexibility, and clinical-to-commercial continuity.

## **Recent Developments**

### **Closing of Private Placement**

On November 5, 2024, we closed a private investment in public equity financing (the “November 2024 Private Placement”) in which we sold 1,835,000 shares of Common Stock at a price of \$50.00 per share and, in lieu of shares of Common Stock, Pre-Funded Warrants to purchase up to an aggregate of 2,165,042 shares of Common Stock, at a purchase price of \$49.999 per pre-funded warrant, to certain institutional accredited investors. The Pre-Funded Warrants are immediately exercisable until exercised in full at a price of \$0.001 per share of common stock. The aggregate gross proceeds to the Company totaled approximately \$200.0 million. Net proceeds, net of commissions and other offering expenses, are expected to total approximately \$189.5 million.

The sale of the shares of Common Stock and and Pre-Funded Warrants in the November 2024 Private Placement was not registered under the Securities Act, and such sale was intended to be exempt from registration pursuant to Section 4(a)(2) of the Securities Act as a transaction by an issuer not involving a public offering.

### **Corporation Information**

On December 18, 2023 (the “Closing”), we completed our business combination with Neurogene Inc., a Nevada corporation (“Neurogene OpCo”) in accordance with the terms of the Agreement and Plan of Merger, dated as of July 17, 2023 (the “Merger Agreement”), by and among the Company, Project North Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of the Company (“Merger Sub”), and Neurogene OpCo, pursuant to which, among other matters, Merger Sub merged with and into Neurogene OpCo, with Neurogene OpCo surviving as a wholly owned subsidiary of the Company (the “Reverse Merger”). In connection with the completion of the Reverse Merger, the Company changed its name from “Neoleukin Therapeutics, Inc.” to “Neurogene Inc.,” and the business conducted by the Company became primarily the business conducted by Neurogene OpCo. Immediately prior to Closing, the Company effected a 1-for-4 reverse stock split (the “Reverse Stock Split”). Unless noted otherwise, all references in this prospectus to share and per share amounts reflect the Reverse Stock Split.

Concurrently with the execution and delivery of the Merger Agreement, and in order to provide Neurogene OpCo with additional capital for its development programs, Neurogene OpCo entered into a subscription agreement (the “Subscription Agreement”) with certain investors named therein (the “Investors”), pursuant to which, subject to the terms and conditions of the Subscription Agreement, immediately prior to the effective time of the Reverse Merger, Neurogene OpCo issued and sold, and the Investors purchased, 2,792,206 shares of Neurogene OpCo common stock and 1,811,739 pre-funded warrants, exercisable for 1,811,739 shares of Neurogene OpCo common stock, at a purchase price of approximately \$20.63 per share or \$20.63 per warrant, for an aggregate purchase price of approximately \$95.0 million (the “Pre-Closing Financing”).

We make available free of charge on our website our annual report on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports as soon as reasonably practicable after we electronically file or furnish such materials to the SEC. The reports are also available at [www.sec.gov](http://www.sec.gov). Our website address is [www.neurogene.com](http://www.neurogene.com). The information contained on, or that can be accessed through, our website is not part of, and is not incorporated by reference into, this prospectus.

### **Implications of Being a Smaller Reporting Company**

We are a “smaller reporting company,” meaning that the market value of our Common Stock held by non-affiliates is less than \$700.0 million and our annual revenue is less than \$100.0 million during the most recently completed fiscal year. We will continue to be a smaller reporting company so long as either (i) the market value of our Common Stock held by non-affiliates is less than \$250.0 million or (ii) our annual revenue is less than \$100.0 million during the most recently completed fiscal year and the market value of our Common Stock held by non-affiliates is less than \$700.0 million. As a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and smaller reporting companies have reduced disclosure obligations regarding executive compensation.

**The Offering**

Common Stock to be Offered by the Selling Stockholders	4,000,042 shares, which includes 2,165,042 shares of common stock issuable upon the exercise of pre-funded warrants held by certain of the Selling Stockholders.
Terms of the Offering	The Selling Stockholders will determine when and how they will dispose of the Resale Shares registered under this prospectus for resale.
Use of Proceeds	We will not receive any proceeds from the sale of the shares of common stock covered by this prospectus. The net proceeds from the sale of the Resale Shares offered by this prospectus will be received by the Selling Stockholders. Upon any exercise of the Pre-Funded Warrants by payment of cash, however, we will receive the nominal cash exercise price paid by the holders of the Pre-Funded Warrants. See the section titled "Use of Proceeds."
Risk Factors	See " <a href="#">Risk Factors</a> " beginning on page 8, for a discussion of factors you should carefully consider before deciding to invest in our common stock.
NASDAQ Global Market symbol	NGNE

## RISK FACTOR SUMMARY

Investing in shares of our common stock involves a high degree of risk. You should carefully consider the following risks and uncertainties, together with all of the other information contained in this prospectus before making an investment decision. The occurrence of any of the following risks could materially and adversely affect our business, financial condition, reputation, or results of operations. In such case, the trading price of shares of our common stock could decline, and you may lose all or part of your investment. It is not possible to predict or identify all such risks; our operations could also be affected by factors, events or uncertainties that are not presently known to us or that we currently do not consider to present significant risks to our operations. Therefore, you should not consider the following risks to be a complete statement of all the potential risks or uncertainties that we face.

### Summary of Risk Factors

- We have a limited operating history, have not completed any clinical trials, and have no products approved for commercial sale, and our results may vary from quarter to quarter.
- We will require substantial additional capital to finance our operations in the future. If we are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce or eliminate clinical trials, product development programs or future commercialization efforts.
- We have incurred significant losses since inception, expect to incur significant losses for the foreseeable future and may not be able to achieve or sustain profitability in the future. We have no products for sale, have not generated any product revenue and may never generate product revenue or become profitable.
- NGN-401 and our other programs are in early stages of development and may fail in development or suffer delays that materially and adversely affect their commercial viability. If we or our current or future collaborators are unable to complete development of, or commercialize, our product candidates, or experience significant delays in doing so, our business will be materially harmed.
- We are substantially dependent on the success of our most advanced product candidate, NGN-401, and our ongoing and anticipated clinical trials of NGN-401 may not be successful.
- Delays in developing our manufacturing capabilities or failure to achieve operating efficiencies from such capabilities may require us to devote additional resources and management time to manufacturing operations and may delay our product development timelines.
- We have a number of academic collaborations, and currently rely on our collaboration with the University Court of the University of Edinburgh (the “University of Edinburgh”) for certain aspects of our preclinical research and development programs, including working in collaboration to discover and preclinically develop potential product candidates for our near-term future pipeline. Failure or delay of the University of Edinburgh or any other collaborator to fulfil all or part of its obligations under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of the relationship would materially harm our business.
- In order to successfully implement our plans and strategies, we will need to grow the size of our organization and we may experience difficulties in managing this growth.
- The regulatory approval processes of the U.S. Food and Drug Administration (“FDA”) and other comparable foreign regulatory authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for our product candidates, we will not be able to commercialize, or will be delayed in commercializing, such product candidates, and our ability to generate revenue will be materially impaired.
- The market price of our common stock may continue to be volatile.

- We may be required to allocate resources to fulfilling the requirements of the Contingent Value Rights Agreement entered into in connection with the Reverse Merger related to certain legacy lease obligations which may take away from our core programs and create a distraction for our management and employees.
- Future sales of shares by existing stockholders could cause our stock price to decline.
- Our executive officers, directors and principal stockholders have the ability to control or significantly influence all matters submitted to our stockholders for approval.

## RISK FACTORS

### Risks Related to Our Limited Operating History, Financial Position and Capital Requirements

*We have a limited operating history, have not completed any clinical trials, and have no products approved for commercial sale, and our results may vary from quarter to quarter.*

We are a clinical-stage biotechnology company with limited operating history. Since our inception in 2018, we have incurred significant operating losses and have used substantially all of our resources to conduct research and development activities, preclinical studies and Phase 1/2 clinical trials of our most advanced product candidates, establish in-house manufacturing capabilities, including analytical and process development operations to support ongoing manufacturing operations, manufacture product candidates, conduct business planning, develop and maintain our intellectual property portfolio, hire personnel, raise capital, and provide general and administrative support for these activities. We have limited experience as a company in initiating, conducting or completing clinical trials. In part because of this lack of experience, we cannot be certain that our current and planned clinical trials will begin on time, meet our anticipated timelines for enrollment and data analysis, or be completed on time, if at all. In addition, while we are conducting a Phase 1/2 clinical trial of NGN-401 in patients with Rett syndrome and have completed enrollment in a Phase 1/2 clinical trial of NGN-101 in patients with CLN5 Batten disease, we have not yet demonstrated our ability to successfully complete clinical trials (including Phase 3 or other pivotal clinical trials), obtain regulatory or marketing approvals, manufacture a commercial-scale product or arrange for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization. Additionally, we expect our financial condition and operating results to continue to fluctuate significantly from period to period due to a variety of factors, many of which are beyond our control. Consequently, any predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as our business grows, we may encounter unforeseen expenses, restrictions, difficulties, complications, delays and other known and unknown factors. We will need to transition at some point from a company with an early research and development focus to a company capable of supporting larger pivotal clinical trials and eventually commercial activities, including the manufacture of commercial scale product. We may not be successful in such a transition.

*We will require substantial additional capital to finance our operations in the future. If we are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce or eliminate clinical trials, product development programs or future commercialization efforts.*

Developing biotechnology products is a long, time-consuming, expensive and uncertain process that takes years to complete. Since our inception, we have funded our operations primarily through private financings and have incurred significant recurring losses, including a cumulative net loss from inception of \$248.2 million. We expect our expenses to increase in connection with our ongoing activities, particularly as we continue to conduct a Phase 1/2 clinical trial of NGN-401 in patients with Rett syndrome, with the expectation that we will also initiate additional clinical trials in the future, and continue to research, develop and conduct preclinical studies of our other potential product candidates. We also anticipate that we may have near term expenses related to NGN-101 as we continue to evaluate options for that program following the denial by the FDA of an RMAT designation, which precludes our ability to use a streamlined registration pathway necessary for further investment in the program.

In addition, if we obtain regulatory approval for any product candidate for commercial sale, including NGN-401, we anticipate incurring significant commercialization expenses related to product manufacturing, marketing, sales and distribution activities to launch any such product. Our expenses could increase beyond expectations if we are required by the FDA or other regulatory agencies to perform preclinical studies or clinical trials in addition to those that we currently anticipate. Because the design and outcome of our current, planned and anticipated clinical trials are highly uncertain, we cannot reasonably estimate the actual amount of funding that will be necessary to successfully complete the development and commercialization of any product candidate we develop. Our future capital requirements depend on many factors, including factors that are not within our control.

We have incurred and expect to continue to incur additional costs associated with operating as a public company, and we do not anticipate achieving any significant revenue in the near term given the development stage of our product candidates. Accordingly, we will require substantial additional funding to continue our operations. Based on our current operating plan, after giving effect to our private placement financing in November 2024, we believe that our existing cash, cash equivalents and short-term investments should be sufficient to fund our operations into the second half of 2027. This estimate is based on assumptions that may prove to be materially wrong, and we could deplete our available capital resources sooner than we currently expect. Our future capital requirements will depend on many factors, including:

- the timing and progress of preclinical and clinical development activities, including any impact to our NGN-401 clinical trial activities relating to our participation in the FDA’s Support for clinical Trials Advancing Rare disease Therapeutics (“START”) program and the Regenerative Medicine Advanced Therapy (“RMAT”) program;
- the number and scope of preclinical and clinical programs we pursue to develop our gene therapy candidate pipeline and EXACT (Expression Attenuation via Construct Timing) platform;
- our ability to secure appropriate animal models for the conduct of investigational new drug (“IND”)-enabling studies in a timely and financially feasible manner, especially large animal models, such as non-human primates (“NHPs”) needed for toxicology studies;
- our ability to establish an acceptable safety profile with IND-enabling toxicology studies to enable clinical trials;
- successful patient enrollment in, and the initiation and completion of, larger and later-stage clinical trials;
- the number of subjects that participate in clinical trials and per subject trial costs;
- the number and extent of trials required for regulatory approval;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible subjects in clinical trials;
- the drop-out and discontinuation rate of subjects;
- potential additional safety monitoring requested by regulatory agencies;
- the duration of subject participation in the trials and follow-up;
- the extent to which we encounter any serious adverse events in our clinical trials;
- the timing of receipt of regulatory approvals from applicable regulatory authorities, including those required to initiate clinical trials;
- the timing, receipt and terms of any marketing approvals and post-marketing approval commitments from applicable regulatory authorities;
- the extent to which we establish collaborations, strategic partnerships, or other strategic arrangements with third parties, if any, and the performance of any such third party;
- the scale up of our clinical and regulatory capabilities, including establishing our current good manufacturing practices (“cGMP”) manufacturing capabilities to support expansion of our pipeline and future registration-enabling clinical trials, and obtaining cGMP material for clinical trials or potential commercial sales;
- hiring and retaining research, clinical, regulatory, manufacturing (including quality control and quality assurance) and administrative personnel;

- our arrangements with third-party contract development and manufacturing organizations (“CDMOs”) and contract research organizations (“CROs”);
- the outfitting and validation of our cGMP manufacturing facility, including expansion to commercial scale;
- the impact of any business interruptions to our operations or to those of the third parties with whom we work; and
- obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights.

We do not have any committed external sources of funds, and adequate additional financing may not be available to us on acceptable terms, or at all. We may be required to seek additional funds sooner than planned through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. Such financing may dilute our stockholders or the failure to obtain such financing may restrict our operating activities. Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our business. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences and anti-dilution protections that adversely affect your rights as a stockholder. Debt financing may result in imposition of debt covenants, increased fixed payment obligations or other restrictions that may affect our business. If we raise additional funds through upfront payments or milestone payments pursuant to future collaborations with third parties, we may have to relinquish valuable rights to product development programs, or grant licenses on terms that are not favorable to us. Our ability to raise additional capital may be adversely impacted by global macroeconomic conditions, geopolitical instability, changes in government regulations and volatility in the credit and financial markets in the United States and worldwide, over which we may have no or little control. Our failure to raise capital as and when needed or on acceptable terms would have a negative impact on our financial condition and our ability to pursue our business strategy, and we may have to delay, reduce the scope of, suspend or eliminate clinical trials, product development programs or future commercialization efforts.

***We have incurred significant losses since inception, expect to incur significant losses for the foreseeable future and may not be able to achieve or sustain profitability in the future. We have no products for sale, have not generated any product revenue and may never generate product revenue or become profitable.***

Investment in biotechnology product development is a highly speculative undertaking and entails substantial upfront expenditures and significant risks that any program will fail to demonstrate adequate efficacy or potency or an acceptable safety profile, gain regulatory approval and become commercially viable. We have no products approved for commercial sale, have not generated any revenue from product sales to date, and continue to incur significant research and development and other expenses related to our ongoing operations. We do not expect to generate product revenue unless or until we successfully complete clinical development and obtain regulatory approval of, and then successfully commercialize, at least one product candidate. We may never succeed in these activities and, even if we do, we may never generate product revenue or revenues that are significant or large enough to achieve profitability. If we are unable to generate sufficient revenue through the sale of any approved products, we may be unable to continue operations without additional funding.

We have incurred significant net losses in each period since we commenced operations in 2018. Our net loss was \$55.6 million for the nine months ended September 30, 2024 and our cumulative net loss from inception as of September 30, 2024 was \$242.8 million. We expect to continue to incur significant losses for the foreseeable future. Our operating expenses and net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially if and as we:

- advance our existing and future programs through preclinical and clinical development, including expansion into additional indications;
- seek to identify additional programs and additional product candidates;
- continue to develop our gene therapy product candidate pipeline and our EXACT platform;



- maintain, expand, enforce, defend and protect our intellectual property portfolio;
- seek regulatory and marketing approvals for product candidates;
- seek to identify, establish and maintain additional collaborations and license agreements, including those which may enhance the biodistribution and delivery of our product candidates;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any biological products for which we may obtain marketing approval, either by ourselves or in collaboration with others;
- generate revenue from commercial sales of products for which we receive marketing approval;
- hire additional personnel, including research and development, clinical and commercial;
- add operational, financial and management information systems and personnel to support further expansion and operation as a public company;
- acquire or in-license products, intellectual property and technologies which may enhance our current technology; and
- establish commercial-scale cGMP capabilities through our own or third-party manufacturing facilities.

In addition, our expenses will increase if, among other things, we are required by the FDA or other regulatory authorities to perform trials or studies in addition to, or different than, those that we currently anticipate, there are any delays in completing our clinical trials or the development of any product candidates, or there are any third-party challenges to our intellectual property or we need to defend against any intellectual property-related claim.

Even if we obtain marketing approval for, and are successful in commercializing, one or more product candidates, we expect to incur substantial additional research and development and other expenditures to develop and market additional programs and/or to expand the approved indications of any marketed product. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue.

Our failure to become profitable would decrease our value and could impair our ability to raise capital, maintain our research and development efforts, expand our business and/or continue our operations. A decline in our value could also cause you to lose all or part of your investment.

#### **Risks Related to Discovery, Development and Commercialization**

***We face competition from entities that have developed or may develop programs for the diseases we plan to address with NGN-401 and other product candidates in development.***

The development and commercialization of biological products is highly competitive. If approved, NGN-401 or any other product candidates we may develop will face significant competition and our failure to effectively compete may prevent us from achieving significant market penetration. We compete with a variety of multinational biopharmaceutical companies, specialized biotechnology companies and emerging biotechnology companies, as well as academic institutions, governmental agencies, and public and private research institutions, among others. Many of the companies with which we are currently competing or will compete against in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industry may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, NGN-401 and any other product candidates we may develop.

As described in “*Business—Competition*” in our Annual Report on Form 10-K, our competitors have developed, are developing or may develop programs or clinical stage products competitive with NGN-401 or our other earlier stage product candidates. Competitive therapeutic treatments include those that have already been approved and accepted by the medical community for Rett syndrome and any new treatments for Rett syndrome. Our success will depend partially on our ability to develop and commercialize products that have a competitive safety, efficacy or potency, dosing and/or presentation profile. Our commercial opportunity and success will be reduced or eliminated if competing products are safer, more effective or potent, have a more attractive or less invasive dosing profile or presentation or are less expensive than any products we may develop, or if competitors develop competing products that enter the market more quickly than we are able to, if we are able to at all, and are able to gain market acceptance.

***NGN-401 and our other preclinical programs are in early stages of development and may fail in development or suffer delays that materially and adversely affect their commercial viability. If we or our current or future collaborators are unable to complete development of, or commercialize, our product candidates, or experience significant delays in doing so, our business will be materially harmed.***

We have no products on the market. NGN-401 is in the early stages of clinical development. Also, we recently announced that unless we are able to find an alternative pathway for advancement, we will need to discontinue our NGN-101 program following the denial of RMAT designation for NGN-101 by the FDA which would preclude a streamlined path to regulatory approval.

We also anticipate that we may have near term expenses related to NGN-101 as we continue to evaluate options for that program following the denial by the FDA of an RMAT designation, which precludes our ability to use a streamlined registrational pathway necessary for further investment in the program.

Our other programs are in early stages of preclinical development. As a result, we expect it will be many years before we commercialize our product candidates and ultimately may not be successful in commercializing any of our product candidates. Our ability to achieve and sustain profitability depends on obtaining regulatory approvals for, and successfully commercializing, our lead product candidate NGN-401 or other product candidates, either alone or with third parties, and we cannot guarantee that we will ever obtain regulatory approval for any product candidates we may develop.

We have limited experience as a company in conducting and managing the clinical trials necessary to obtain regulatory approvals, including approval by the FDA or comparable foreign regulatory authorities. We have not yet demonstrated our ability to obtain regulatory approvals, manufacture a commercial scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Before obtaining regulatory approval for the commercial distribution of product candidates, we or an existing or future collaborator must conduct extensive preclinical tests and clinical trials to demonstrate the safety, purity and efficacy or potency in humans of such product candidates.

In November 2024, we announced that we do not expect to move forward with the NGN-101 gene therapy program for CLN5 Batten disease at this time. Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. Similar challenges may prevent our success with or increase the cost of other current, planned or future clinical trials. We or our collaborators may experience delays in initiating or completing clinical trials, and also may experience unforeseen events during, or as a result of, any current or future clinical trials that could delay or prevent our ability to receive marketing approval or commercialize NGN-401 or any other product candidates, including:

- regulators or institutional review boards (“IRBs”), the FDA or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or may fail to reach, agreement on acceptable terms with prospective trial sites and prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;

- the observation of an actual or suspected unexpected serious adverse reaction, serious adverse events, or adverse events of special interest could result in a partial or complete clinical hold for an unpredictable length of time, delay or halt future enrollment, require increased staggering between patient dosing, require dose reductions that could adversely affect the anticipated efficacy or potency product profile, or require a program discontinuation;
- clinical trial sites may fail to meet enrollment targets, may deviate from trial protocol, or may experience patients dropping out of a trial;
- clinical trials of any product candidates may fail to show safety or efficacy or potency, or produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical studies or clinical trials or we may decide to abandon product development programs;
- the number of subjects required for clinical trials of any of our product candidates may be larger than we anticipate, especially if the effect size observed in future clinical data from a Phase 1/2 clinical trial is small or is difficult to ascertain relative to natural history as a comparator, or if regulatory authorities require completion of a sham-controlled clinical trial;
- enrollment in clinical trials may be slower than we anticipate or subjects may drop out of clinical trials or fail to return for post-treatment follow-up at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or may deviate from the clinical trial protocol or drop out of the trial, which may require that we add new clinical trial sites or investigators;
- we may elect to, or regulators, independent data and safety monitoring boards (“DSMBs”), IRBs or ethics committees may require that we or our investigators suspend or terminate clinical research or trials, or delay further dosing of subjects in clinical trials, for various reasons, including noncompliance with regulatory requirements or a finding that the participants in our trials are being exposed to unacceptable health risks;
- the cost of clinical trials of any of our product candidates may be greater than we anticipate;
- the quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be inadequate to initiate or complete a given clinical trial;
- we may be unable to manufacture sufficient quantities of our product candidates for use in clinical trials;
- reports from clinical testing of other therapies may raise safety, efficacy or potency concerns about our product candidates;
- we may fail to establish an appropriate safety profile for a product candidate based on clinical or preclinical data for such product candidate and data emerging from other therapies in the same class as our product candidates; and
- the FDA or other regulatory authorities may require us to submit additional data, such as long-term toxicology studies, or impose other requirements before permitting us to initiate a clinical trial.

If safety concerns develop with respect to our product candidates or clinical trial designs, we may be delayed in our development plans as we may need to pause our enrollment in a clinical trial, revise our trial designs, or take other measures that may increase the amount of time and resources required to bring our product candidates forward. For example, on November 11, 2024, we were advised of a serious adverse effect (“SAE”) experienced by a participant in the 3E15 vg (high dose) cohort of our Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome. The participant subsequently died following complications from a rare and life-threatening hyperinflammatory syndrome associated with systemic exposure to high doses of AAV. The FDA completed a review of the safety data for NGN-401 and allowed us to continue with the Phase 1/2 trial using the 1E15 vg dose (low-dose cohort), although we will need to revise our trial protocol before we resume dosing. We paused further

use of the 3E15 vg dose (high dose cohorts) upon initial notification of the SAE and made the determination to remove that dose level from the trial protocol as we do not plan to enroll any further participants at the 3E15 vg dose level.

Commencing clinical trials in the United States is subject to acceptance by the FDA of an IND or, if commenced in other jurisdictions, acceptance by the comparable foreign regulatory agency of a similar application, as well as finalizing the trial design. In the event that the FDA or applicable foreign regulatory agency requires us to complete additional preclinical studies, or we are required to satisfy other regulatory requests prior to commencing clinical trials, the start of our clinical trials may be delayed. Even after we receive and incorporate guidance from these regulatory authorities, the FDA or other regulatory authorities could disagree that we have satisfied their requirements to commence any clinical trial or change their position on the acceptability of our trial design or the clinical endpoints selected, which may require us to complete additional preclinical studies or clinical trials, delay the enrollment of our clinical trials or impose stricter approval conditions than we currently expect. There are equivalent processes and risks applicable to clinical trial applications in other jurisdictions, including the United Kingdom (“UK”), Australia and the European Union.

We may not have the financial resources to continue development of, or to modify existing collaborations or enter into new collaborations for, a product candidate if we experience any issues that delay or prevent regulatory approval of, or our ability to commercialize, NGN-401 or any other product candidates we are developing or may develop in the future. We or our current or future collaborators’ inability to complete development of, or commercialize, NGN-401 or any other product candidates or significant delays in doing so, could have a material and adverse effect on our business, financial condition, results of operations and prospects.

***We currently utilize adeno-associated virus serotype 9 (“AAV9”) capsid for delivery of therapeutic transgenes to deliver our product candidates, which may limit the safety, purity, and efficacy or potency of such product candidates.***

Our current approach is to identify, develop and commercialize gene therapy product candidates using an AAV9 capsid for delivery of therapeutic transgenes to certain kinds of cells.

Although AAV9 has been tested in numerous clinical trials and is an approved serotype for at least one gene therapy product, we cannot be certain that our AAV9 product candidates will successfully advance through preclinical studies and clinical trials, or that they will not cause significant adverse events or toxicities. In November 2024, a participant recently dosed in the 3E15 vg (high dose) cohort of our Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome experienced an SAE consistent with known risks of AAV gene therapy, and ultimately died from this complication. While this reaction was very rare, we cannot ensure that other SAEs related to the use of AAV9 will not occur, or that we will not experience delays or other negative impacts to our clinical trial related to this or other AAV-related SAEs. We also cannot be certain that we will be able to avoid triggering toxicities in our future preclinical studies or clinical trials or that our chosen routes of administration to deliver such therapies will not cause unforeseen side effects or other challenges. Although AAV9 has been shown to facilitate biodistribution and cell transduction to the central nervous system (“CNS”), the potentially limited levels of AAV9 transduction of cells in the CNS and certain retinal cells may also limit the potential efficacy or potency of any of our product candidates, including NGN-401.

***We intend to identify and develop novel gene therapy product candidates, which makes it difficult to predict the time, cost and potential success of product candidate development.***

A key part of our business strategy is to identify and develop additional product candidates. As such, our future success depends on the successful development of novel therapeutic approaches, including by utilizing our EXACT technology or other transgene regulation technology. Our preclinical research and clinical trials may initially show promise in identifying potential product candidates, yet fail to yield product candidates for a number of reasons. For example, although EXACT is designed to deliver therapeutic levels of transgene while avoiding overexpression toxicity and off-target effects, there can be no assurance that any EXACT transgene regulation will result in product candidates that are shown in clinical trials to be safe, pure, and effective or potent.

To date, very few products that utilize gene transfer have been approved in the United States, Europe or other markets, and no products have been approved using our EXACT technology or technology similar to it. There have been a limited number of clinical trials of gene transfer technologies, with only very few product candidates ever approved by the FDA or comparable foreign regulatory authorities.

As a result, it is difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our approach to gene therapy will result in the identification, development, and regulatory approval of any product candidates, or that other gene therapy programs will not be considered better or more attractive. There can be no assurance that any development problems we experience in the future related to our current gene therapy approaches or product candidates or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Research programs to identify new product candidates require substantial technical, financial, and human resources. If we are unable to identify suitable gene therapy product candidates for preclinical and clinical development, we may not be able to successfully implement our business strategy, and may have to delay, reduce the scope of, suspend or eliminate one or more of our product candidates, clinical trials or future commercialization efforts, which would negatively impact our financial condition.

***The disorders we seek to treat have low prevalence and it may be difficult to identify and enroll patients with these disorders. If we experience delays or difficulties in the enrollment and/or maintenance of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.***

Successful and timely completion of clinical trials will require that we enroll and maintain a sufficient number of patients. Patient enrollment is affected by many factors, including the size and nature of the patient population and competition for patients with other trials. Genetic diseases generally, and especially the rare diseases for which some of our current product candidates are targeted, have low incidence and prevalence. For example, we estimate global incidence of Rett syndrome to be approximately one in 10,000 live female births. Accordingly, it may be difficult for us to identify and timely recruit a sufficient number of eligible patients to conduct our clinical trials. Further, any natural history studies that we or our collaborators may conduct may fail to provide us with patients for our clinical trials because patients enrolled in the natural history studies may not be good candidates for our clinical trials, or may choose to not enroll in our clinical trials.

Trials may be subject to delays as a result of patient enrollment taking longer than anticipated or patient withdrawal. We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA, the European Medicines Agency (“EMA”) or other foreign regulatory authorities. We cannot predict how successful we will be at enrolling subjects in future clinical trials. Subject enrollment is affected by other factors including:

- the eligibility criteria for the trial in question;
- the timely diagnosis of disease to meet such eligibility criteria;
- the size of the patient population and process for identifying patients;
- the perceived risks and benefits of the product candidate in the trial, especially by clinician experts and patient advocacy organizations, including relating to AAV9-based gene therapy and intracerebral spinal fluid delivery system;
- the availability of competing commercially available therapies and other competing therapeutic candidates’ clinical trials;
- the willingness of caregivers to enroll their children in our clinical trials;
- the efforts to facilitate timely enrollment in clinical trials;
- potential disruptions caused by pandemics or other public health crises, including difficulties in initiating clinical sites, enrolling and retaining participants, diversion of healthcare resources away from clinical trials, travel or quarantine policies that may be implemented, and other factors;

- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- the proximity and availability of clinical trial sites for prospective patients.

Even if we are able to enroll a sufficient number of patients in our clinical trials, we may have difficulty maintaining enrollment of such patients. Our inability to enroll or maintain a sufficient number of patients would result in significant delays in completing clinical trials or receipt of marketing approvals and increased development costs, or may require us to abandon one or more clinical trials altogether.

***We are substantially dependent on the success of our most advanced product candidate, NGN-401, and our ongoing and anticipated clinical trials of NGN-401 may not be successful.***

Our future success is substantially dependent on our ability to timely obtain marketing approval for, and then successfully commercialize, our most advanced product candidate, NGN-401. We are investing a majority of our efforts and financial resources into the research and development of this product candidate, as we are currently conducting a Phase 1/2 clinical trial of NGN-401 in patients with Rett syndrome. Based on the initial interim positive clinical data from our 1E15 vg dose of NGN-401 in our Phase 1/2 clinical trial of NGN-401, we expect to advance that product candidate to a registrational clinical trial, pending future regulatory feedback on various aspects of development such as the pivotal trial design and manufacturing related requirements.

NGN-401 will require additional clinical development, evaluation of clinical, preclinical and manufacturing activities, marketing approval in multiple jurisdictions, substantial investment and significant marketing efforts before we generate revenues from product sales, if any. We are not permitted to market or promote this product candidate, or any other product candidates we may develop, before we receive marketing approval from the FDA and/or comparable foreign regulatory authorities, and we may never receive such marketing approvals.

The success of NGN-401 will depend on a variety of factors. We do not have complete control over many of these factors, including certain aspects of clinical development and the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing, distribution and sales efforts of any future collaborator. Accordingly, we cannot guarantee that we will ever be able to generate revenue through the sale of this product candidates, even if approved. If we are not successful in commercializing NGN-401, or are significantly delayed in doing so, our business will be materially harmed.

***We may not be successful in identifying and advancing a strategy to continue the development of NGN-101 for CLN5 Batten disease, and in the meantime, may incur additional costs as we continue the post-dosing phases of our clinical trial for NGN-101.***

In November 2024, we announced that we do not expect to move forward with the NGN-101 CLN5 Batten disease gene therapy program at the present time because of an inability to align on a streamlined registrational pathway with the FDA for that product candidate, but that we would continue to evaluate options for that program. We may consider a range of potential alternatives for the program, which could include continuing to discuss possibilities for a streamlined pathway to registration with the FDA, looking for a partner or out-licensing the product candidate entirely, but there can be no assurance that we will find any alternative to move the program forward.

In addition, while we have completed dosing in the Phase 1/2 clinical trial of NGN-101 for CLN5 Batten disease, we do intend to continue to follow the patients who received treatment in the clinical trial and therefore may continue to incur certain incremental costs related to the continued observations in that clinical trial, even if we are not able to find a future path to commercialization for this product candidate.

***Our programs are focused on the development of therapeutics for patients with neurological diseases, which is a rapidly evolving area of science, and the approach we are taking to discover and develop product candidates is novel and may never lead to approved or marketable products.***

The discovery and development of therapeutics for patients with neurological diseases is an emerging field, and the scientific discoveries that form the basis for our efforts to discover and develop product candidates are relatively new. The scientific evidence to support the feasibility of developing product candidates based on these discoveries is both preliminary and limited. Although we believe, based on our preclinical work, that our programs have the potential to be disease-modifying therapies, clinical results may not confirm this hypothesis or may only confirm it for certain alterations or certain indications. The patient populations for our product candidates are limited to those with specific neurological diseases. We cannot be certain that the patient populations for each specific disease will be large enough to allow us to successfully obtain approval and commercialize our product candidates and achieve profitability. Further, our Phase 1/2 clinical trial of NGN-401 will involve a small patient population. Because of the small sample sizes, the results of this trials may not be indicative of results of future clinical trials.

***If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of NGN-401 or any other product candidates may be delayed and, as a result, our stock price may decline.***

From time to time, we estimate the timing of the anticipated accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials and the submission of regulatory filings. From time to time, we may publicly announce the expected timing of some of these milestones. All of these milestones are and will be based on numerous assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in some cases for reasons beyond our control. If we do not meet these milestones as publicly announced, or at all, the commercialization of NGN-401 or any other product candidates may be delayed or never achieved and, as a result, our stock price may decline.

***Preclinical and clinical development involves a lengthy and expensive process that is subject to delays and uncertain outcomes, and results of earlier studies and trials may not be predictive of future clinical trial results. If our preclinical studies and clinical trials are not sufficient to support regulatory approval of any of our product candidates, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development of such product candidate.***

Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical studies, which are a lengthy, time consuming and expensive process with a high risk of failure. The length of time of such testing may vary substantially according to the type, complexity and novelty of the program, and often can be several years or more per program. Delays associated with programs for which we are conducting preclinical testing and studies may cause us to incur additional operating expenses. For example, we depend on the availability of NHPs to conduct certain preclinical studies that we are required to complete prior to submitting an IND and initiating clinical development. A sustained global shortage of NHPs available for biological product development could cause the cost of obtaining NHPs for our future preclinical studies to increase significantly and result in delays to our development timelines. However, after conducting preclinical studies, we must then conduct extensive clinical trials to demonstrate the safety, purity, and efficacy or potency of our product candidate in humans. Our clinical trials may not be conducted as planned or completed on schedule, if at all.

Furthermore, failure can occur at any time during the preclinical study or clinical trial process, and the outcome of preclinical studies and early-stage clinical trials may not be predictive of the success of later clinical trials, especially as our initial clinical trials do not contain a control arm. In addition, we have designed our initial clinical trials with relatively small cohorts before expanding in size and dosing in subsequent cohorts. If safety issues arise in an early cohort, we may be delayed or prevented from dose escalating or subsequently expanding into larger trial cohorts. For example, on November 11, 2024, we were advised of an SAE experienced by a recently dosed participant in the 3E15 vg (high dose) cohort of our Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome. The participant subsequently died following complications from a rare and life-threatening hyperinflammatory syndrome associated with systemic exposure to high doses of AAV. The FDA completed a review of the safety data for NGN-401 and allowed us to proceed with the Phase 1/2 trial using the 1E15 vg dose (low dose cohort), although we will need to revise our trial protocol before we resume dosing. We paused further use of the 3E15 vg dose (high dose cohorts) upon initial notification of the SAE and made the determination to

remove that dose level from the trial protocol as we do not plan to enroll any further participants at the 3E15 vg dose level.

Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates. Earlier gene therapy clinical trials conducted by others also utilized AAV vectors. However, these studies should not be relied upon as evidence that our planned clinical trials will succeed. In addition, we expect to rely on patients, caregivers and clinicians to provide feedback on measures, which are subjective and inherently difficult to evaluate. These measures can be influenced by factors outside of our control, and can vary widely from day to day for a particular patient, and from patient to patient or caregiver to caregiver and from site to site within a clinical trial.

We cannot be sure that the FDA or comparable foreign regulatory authorities will agree with our clinical development plan. We are conducting a Phase 1/2 clinical trial of NGN-401 in patients with Rett syndrome. If the FDA or comparable regulatory authorities requires us to conduct additional trials or enroll additional patients, our development timelines may be delayed or we may not be able to pursue further development due to such delays. For example, in November 2024, we announced that the Company does not expect to move forward with the NGN-101 for CLN5 Batten disease gene therapy program at this time. Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. We cannot be sure that submission of an IND application, clinical trial application (“CTA”) or similar application will result in the FDA or comparable foreign regulatory authorities, as applicable, allowing clinical trials to begin in a timely manner, if at all. Moreover, even if these trials begin, issues may arise that could cause regulatory authorities to require us to suspend or terminate such clinical trials. Events that may prevent successful or timely initiation or completion of clinical trials include: inability to generate sufficient preclinical, toxicology or other *in vivo* or *in vitro* data to support the initiation or continuation of clinical trials; delays in reaching a consensus with regulatory authorities on study design or implementation of the clinical trials; delays or failure in obtaining regulatory authorization to commence a trial; delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites; delays in identifying, recruiting and training suitable clinical investigators; delays in obtaining required IRB approval at each clinical trial site; difficulties in patient enrollment in our clinical trials for a variety of reasons; delays related to safety concerns; delays in manufacturing, testing, releasing, validating or importing/exporting sufficient stable quantities of our product candidates for use in clinical trials or the inability to do any of the foregoing; failure by our CROs, other third parties or us to adhere to clinical trial protocols; failure to perform in accordance with the FDA’s or any other regulatory authority’s good clinical practices (“GCPs”) or applicable regulatory guidelines in other countries; changes to the clinical trial protocols; clinical sites deviating from trial protocol or dropping out of a trial; changes in regulatory requirements and guidance that require amending or submitting new clinical protocols; selection of clinical endpoints that require prolonged periods of observation or analyses of resulting data; transfer of manufacturing processes to larger-scale facilities operated by a CDMO and delays or failure by our CDMOs or us to make any necessary changes to such manufacturing process; and third parties being unwilling or unable to satisfy their contractual obligations to us.

We could also encounter delays if a clinical trial is placed on clinical hold, suspended or terminated by us, the IRBs of the institutions in which such trials are being conducted, or the FDA, the competent authorities and/or ethics committees of the UK, Australia, EU Member States or other regulatory authorities, if a clinical trial is recommended for suspension or termination by the DSMB or equivalent body for such trial, or on account of changes to federal, state, or local laws. If we are required to conduct additional clinical trials or other testing of NGN-401 or any other product candidates beyond those that we contemplate, if we are unable to successfully complete clinical trials of NGN-401 or any other product candidates, if the results of such trials are not positive or are only moderately positive or if there are safety concerns, our business and results of operations may be adversely affected and we may incur significant additional costs.

In addition, even if we are able to successfully complete the clinical trial for NGN-401, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required



before we submit our product candidates for approval. This is particularly true for clinical trials in very rare diseases, such as with our Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome, where the very small patient population makes it difficult to conduct two traditional, adequate and well-controlled studies. In such cases, the FDA or comparable foreign regulatory authorities are often required or permitted to exercise flexibility in approving therapies for such diseases, but obtaining flexibility is uncertain and may never occur. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in the other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or applicable regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates.

***Preliminary, “topline” or interim data from our preclinical studies and clinical trials that we announce or publish from time to time may change as more patient data becomes available and are subject to audit and verification procedures.***

From time to time, we may publicly disclose preliminary, interim or topline data from our preclinical studies and clinical trials, which are based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data. We also make assumptions, estimations, calculations and conclusions as part of our analyses of these data without the opportunity to fully and carefully evaluate complete data. Preliminary, interim or topline results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data previously disclosed. These preliminary, interim or topline data are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available or as patients from our clinical trials continue other treatments. For example, in June 2024, we announced initial safety data related to our dosing of our first four participants in the 3E15 vg dose cohort of our Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome, which suggested a favorable safety profile for the 3E15 vg cohort. In November 2024, an SAE was reported in the 3E15 vg cohort, which caused us to revise our assumptions regarding the safety profile of the 3E15 vg dose. Because of this potential for change, preliminary, interim and topline data should be viewed with caution until final data are available. Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular product candidate, the approvability or commercialization of a particular product candidate and our company in general. In addition, the information we choose to publicly disclose regarding a particular preclinical study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. If the preliminary, interim or topline data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, NGN-401 or any other product candidate may be harmed, which could harm our business, operating results, prospects or financial condition. In addition, differences between preliminary, interim or topline data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

***Our current or future clinical trials may reveal significant adverse events or undesirable side effects not seen in our preclinical studies and may result in a safety profile that could halt clinical development, inhibit regulatory approval or limit commercial potential or market acceptance of NGN-401 or any other product candidates or result in potential product liability claims.***

Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects, adverse events or unexpected characteristics. The 1E15 vg (low dose) cohort of our Phase 1/2 clinical trial for NGN-401 has not shown any such characteristics to date, however, we have not yet completed those clinical trials and in November 2024, a participant who had recently received the 3E15 vg (high dose) cohort of the NGN-401 clinical trial experienced an SAE consistent with known risks of AAV gene therapy and subsequently died following complications from a rare and life-threatening hyperinflammatory syndrome associated with systemic exposure to high doses of AAV. If additional SAEs or other adverse events or other side effects are observed in any of our current or future clinical trials, we may have difficulty recruiting patients to such trials, patients may drop out of our trials, patients may be harmed, or we may be required to delay enrollment or abandon one or more cohorts of a trial

or delay or abandon the trials or our development efforts of one or more product candidates altogether, including NGN-401. We, the FDA, EMA, or other applicable regulatory authorities, or an IRB, may require suspension of any clinical trials of NGN-401 or any other product candidates at any time for various reasons, including a finding that subjects or patients in such trials are being exposed to unacceptable health risks or adverse side effects. Some potential products developed in the biotechnology industry that initially showed therapeutic promise in early-stage trials have later been found to cause side effects that prevented their further development. Even if the side effects do not preclude a product candidate from obtaining or maintaining marketing approval, undesirable side effects may inhibit market acceptance of an approved product due to its tolerability versus other therapies. In addition, as gene replacement has a potentially life-long activity, with no ability to withdraw the product as with other treatment modalities, this profile could prolong the duration of undesirable side effects, which could also inhibit market acceptance. Treatment-emergent adverse events could also affect patient recruitment or the ability of enrolled subjects to complete our clinical trials or could result in potential product liability claims. Potential side effects associated with NGN-401 or any other product candidates may not be appropriately recognized or managed by the treating medical staff, as toxicities resulting from NGN-401 or any other product candidates may not be normally encountered in the general patient population and by medical personnel. Any of these occurrences could harm our business, financial condition, results of operations and prospects significantly.

In addition, even if we successfully advance NGN-401 or any other product candidates through clinical trials, such trials will only include a limited number of patients and limited duration of follow up to such product candidates. As a result, we cannot be assured that adverse effects of NGN-401 or any other product candidates will not be uncovered when a significantly larger number of patients are exposed to such product candidate after approval, or a significantly longer follow up post-dosing is obtained as part of regulators' recommendations for long-term follow up of clinical study subjects treated with gene therapy. Further, any clinical trials may not be sufficient to determine the effect and safety consequences of using our product candidates over a multi-year period.

We have expended substantial efforts and costs testing our EXACT technology in preclinical studies of NGN-401, including completing toxicology studies prior to the FDA providing clearance of the IND for NGN-401. However, we cannot guarantee that significant adverse effects will not be seen in clinical trials for NGN-401, which could result in clinical holds, delays, suspension or withdrawal of our IND. If any of the foregoing events occur or if NGN-401 or NGN-101 or any other product candidates prove to be unsafe, our entire pipeline could be affected, which would have a material adverse effect on our business, financial condition, results of operations and prospects.

***We may expend our limited resources to pursue a particular product candidate, such as NGN-401, and fail to capitalize on candidates that may be more profitable or for which there is a greater likelihood of success.***

Because we have limited financial and managerial resources, we intend to focus our research and development efforts on certain selected product candidates. For example, to date we have allocated significant resources to our most advanced product candidates, NGN-401 and NGN-101. As a result, we may forgo or delay pursuit of opportunities with other potential candidates that may later prove to have greater commercial potential. For example, in November 2024, we announced that the Company does not expect to move forward with NGN-101 CLN5 Batten disease gene therapy program at this time. Given the rarity of the disease, continued investment in the program was predicated on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT designation was denied. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for specific indications may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such candidate.

***Even if regulatory approval is obtained, any approved products resulting from NGN-401 or any other product candidate may not achieve adequate market acceptance among clinicians, patients, healthcare third-party payors and others in the medical community necessary for commercial success and we may not generate any future revenue from the sale or licensing of such products.***

Even if regulatory approval is obtained for NGN-401 or any other product candidates, our product candidates may not gain market acceptance among physicians, patients, healthcare payors or the medical community. We may not generate or sustain revenue from sales of the product due to factors such as whether the product can be sold at a competitive cost and whether it will otherwise be accepted in the market. There is currently one FDA-approved product and multiple other product candidates in various stages of development for the treatment of Rett syndrome. Market participants with significant influence over acceptance of new treatments, such as clinicians and third-party payors, may not adopt a gene therapy replacement with a target product profile such as that of NGN-401 or for its targeted indications, and we may not be able to convince the medical community and third-party payors to accept and use, or to provide favorable reimbursement for, any product candidates developed by us or our existing or future collaborators. Market acceptance of NGN-401 or any other product candidates will depend on many factors, including factors that are not within our control.

Sales of biological products also depend on the willingness of clinicians to prescribe the treatment. We cannot predict whether clinicians, clinicians' organizations, hospitals, other healthcare providers, government agencies or private insurers will determine that any of our approved products are safe, therapeutically effective or potent, cost effective or less burdensome as compared with competing treatments. If NGN-401 or NGN-101 or any other product candidate is approved but does not achieve an adequate level of acceptance by such parties, we may not generate or derive sufficient revenue from that product and may not become or remain profitable.

***We have never commercialized a product candidate and may lack the necessary expertise, personnel and resources to successfully commercialize a product candidate on our own or together with suitable collaborators.***

We have never commercialized a product candidate and currently have no sales force, marketing or distribution capabilities. To achieve commercial success for a product candidate, we may opt to license such product candidate to others, in which case we may rely on the assistance and guidance of our collaborators on that license arrangement. For a product candidate for which we retain commercialization rights and marketing approval, we will have to develop our own sales, marketing and supply organization or outsource these activities to a third party. Factors that may affect our ability to commercialize a product candidate, if approved, on our own include recruiting and retaining adequate numbers of effective sales and marketing personnel, developing adequate educational and marketing programs to increase public acceptance of our approved product candidate, ensuring regulatory compliance of our company, employees and third parties under applicable healthcare laws and other unforeseen costs associated with creating an independent sales and marketing organization. Developing a sales and marketing organization will be expensive and time-consuming and could delay the launch of a product candidate upon approval. Moreover, we may not be able to build an effective sales and marketing organization. If we are unable to build our own distribution and marketing capabilities or to find suitable partners for the commercialization of an approved product candidate, we may not generate revenues from them or be able to reach or sustain profitability.

***We have never completed any late-stage clinical trials and may not be able to file an IND application or other applications for regulatory approval to commence additional clinical trials on the timelines we expect. Even if we are able to complete such trials, the FDA or comparable foreign regulatory authorities may not permit us to proceed or could suspend or terminate any such trial after it has been initiated.***

We are early in our development efforts and will need to successfully complete later-stage and pivotal clinical trials in order to obtain FDA or comparable foreign regulatory approval to market our product candidates. Carrying out clinical trials and the submission of a successful IND or CTA is a complicated process. Even though our product candidate NGN-401 for Rett syndrome has been accepted into the FDA's START program and RMAT program, the combination of which is expected to allow access to frequent advice from FDA staff, intensive guidance on efficient drug development and eligibility for an Accelerated Approval pathway and Priority Review, our lack of experience with FDA submissions may still slow our progress towards FDA approval. We have not yet completed a Phase 1/2 clinical trial and have limited experience as a company in preparing, submitting and prosecuting regulatory filings.

Based on the initial positive interim data from our 1E15 vg dose of NGN-401 in our Phase 1/2 clinical trial of NGN-401, we intend to engage with the FDA and other comparable foreign regulators to determine the requirements to support initiation of a pivotal clinical trial. However, regulatory authorities may recommend changes to the study design for NGN-401, including the number and size of registrational clinical trials required to be conducted in that program. In addition, regulatory authorities could require manufacturing changes or have us implement additional analytical processes prior to initiation of a future clinical trial. Consequently, we may be unable to successfully and efficiently execute and complete necessary clinical trials in a way that leads to regulatory submission and approval of our product candidates, or we may determine that the regulatory requirements for submission are too burdensome to support continued development of one or more of our product candidates, as we did with our NGN-101 product candidate for CLN5 Batten disease, which we plan to terminate due to a lack of alignment with the FDA on a streamlined pathway to registration. Additionally, even if regulatory authorities agree with the design and implementation of the clinical trials set forth in a regulatory meeting, such regulatory authorities may change their requirements in the future. The FDA or comparable foreign regulatory authorities may require the analysis of data from trials assessing different doses of the product candidate alone or in combination with other therapies to justify the selected dose prior to the initiation of large trials in a specific indication. Any delays or failure to initiate clinical trials or obtain regulatory approvals for our trials may prevent us from completing our clinical trials or commercializing our products on a timely basis, if at all. We are subject to similar risks related to the review and authorization of our protocols and amendments by comparable foreign regulatory authorities.

For our preclinical pipeline, if the IND-enabling studies support a decision to advance into clinical development, we would plan to submit an IND or CTA with a foreign regulatory authority. We may not be able to file the IND or CTA in accordance with our desired timelines for future product candidates. For example, we may experience manufacturing delays or other delays with IND-enabling studies, including with suppliers, study sites, or third-party contractors and vendors on which we depend. Moreover, we cannot be sure that submission of an IND application will result in the FDA or comparable foreign regulatory authorities allowing further clinical trials to begin, or that, once begun, issues will not arise that lead us to suspend or terminate such clinical trials.

### **Risks Related to Manufacturing**

***Gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.***

The manufacture of gene therapy products is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies. While we are currently establishing our own manufacturing facility to provide clinical and commercial supply of our product candidates, we expect to rely on contract manufacturers for certain portions of our manufacturing needs for the foreseeable future, such as those related to research grade material for our early preclinical studies.

The manufacturers of biological and pharmaceutical products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure of us or our CDMOs to adhere to or document compliance with such regulatory requirements could lead to a delay or interruption in the availability of our program materials for clinical trials or enforcement action from the FDA, EMA or other foreign regulatory authorities. If we or our manufacturers were to fail to comply with the FDA, EMA or other regulatory authority, it could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates. Our potential future dependence upon others for the manufacture of our product candidates may also adversely affect our future profit margins and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

Biological products are inherently difficult to manufacture. Although we believe that the manufacture of our product candidates may be simplified due to their shared raw materials and other similarities, we cannot be certain that this will be the case and we may be required to develop manufacturing methods that ultimately differ

significantly between product candidates, which would require that we invest substantial time and capital to develop suitable manufacturing methods. Our program materials are manufactured using technically complex processes requiring specialized equipment and facilities, highly specific raw materials, cells, and reagents, and other production constraints. Our production process requires a number of highly specific raw materials, cells and reagents with limited suppliers. Even though we aim to have backup supplies of raw materials, cells and reagents whenever possible, we cannot be certain those supplies will be sufficient if our primary sources are unavailable. One or more of our suppliers is the sole source of certain materials used by us in our manufacturing process, and a disruption of the supply of those materials could also negatively impact our ability to manufacture clinical supply as we would have to suspend or revise our operations to accommodate for any disruption in the supply of those materials. A shortage of a critical raw material, cell line, or reagent, or a technical issue during manufacturing, may lead to delays in clinical development or commercialization plans. We are particularly susceptible to any shortages, delays or inability to obtain suitable raw materials, given that all of our current and planned product candidates require this starting material. Any changes in the manufacturing of components of the raw materials we use could result in unanticipated or unfavorable effects in our manufacturing processes, resulting in delays.

Once the biological products are manufactured, the product must be analyzed utilizing assays and meet pre-determined specifications in order to be used in certain preclinical studies, in any clinical trial, and, if approval is obtained, for commercial distribution. This testing is performed in-house and at third-party contract manufacturers. Delays or other unexpected obstacles in developing analytical methods or in performing the tests and obtaining the results in-house or at a third-party contractor could result in unanticipated impact to our ability to supply material as needed for pre-clinical, clinical, or commercial needs.

***We and our contract manufacturers for AAV9 are subject to significant regulation with respect to manufacturing of our products. The third-party manufacturing facilities on which we rely, our in-house manufacturing facility, and any manufacturing facility that we may have in the future, may have limited capacity or fail to meet the applicable stringent regulatory requirements.***

We currently have relationships with a limited number of suppliers for the raw materials, including plasmids and virus banks, required by the manufacturing processes of our product candidates. Virus intended for use in our early preclinical studies has been and can be externally supplied; however, if we experience slowdowns or problems with our in-house manufacturing facility and are unable to establish or scale our internal manufacturing capabilities, we will need to continue to contract with manufacturers to produce the preclinical, clinical and commercial supply and such supply will be more uncertain and subject to delays. In addition, each supplier may require licenses to manufacture certain components of the supply if such processes are not owned by the supplier or in the public domain and we may be unable to license such intellectual property rights on reasonable commercial terms or to transfer or sublicense the intellectual property rights we may have with respect to such activities.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for components of our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including recordkeeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a biologics license application (“BLA”) or marketing authorization application (“MAA”) on a timely basis. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our current or future product candidates. In addition, regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our current or future product candidates or the associated quality systems for compliance with the regulations applicable to the activities being conducted, and they could put a hold on one or more of our clinical trials if the facilities of our CDMOs do not pass such audit or inspections. If these facilities do not pass a pre-approval plant inspection, the FDA or other foreign regulatory agency approval of the products will not be granted.

Regulatory authorities also may, at any time following approval of a product for sale, inspect or audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement, and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could harm our business. If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA or other foreign regulatory agencies can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be harmed.

Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. An alternative manufacturer would need to be qualified through a BLA and/or MAA supplement, which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully, if approved. Further, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose future potential revenue, if any.

***We depend on third-party suppliers for materials used in the manufacture of our product candidates, and the loss of these third-party suppliers or their inability to supply us with adequate materials could harm our business.***

We rely on third-party suppliers for certain materials and components required for the production of our product candidates. Our dependence on these third-party suppliers and the challenges we may face in obtaining adequate supplies of materials involve several risks, including limited control over pricing, availability and quality of supplies and delivery schedules. There is substantial demand and limited supply for certain of the raw materials used to manufacture gene therapy products. As a small company, our negotiation leverage is limited and we are likely to get lower priority than our larger competitors. We cannot be certain that our suppliers will continue to provide us with the quantities of raw materials that we require or satisfy our anticipated specifications and quality requirements. One or more of our suppliers is the sole source of certain materials used by us in our manufacturing process, and a disruption of the supply of those materials could also negatively impact our ability to manufacture clinical supply as we would have to suspend or revise our operations to accommodate for any disruption in the supply of those materials. Any supply interruption in limited or sole sourced raw materials could materially harm our ability to manufacture our product candidates until a new source of supply, if any, could be identified and qualified. We may be unable to find a sufficient alternative supply channel in a reasonable time or on commercially reasonable terms. Any performance failure on the part of our suppliers could delay the development and potential commercialization of our product candidates, including limiting supplies necessary for clinical trials and regulatory approvals, which would have a material adverse effect on our business.

***Delays in developing our manufacturing capabilities or failure to achieve operating efficiencies from such capabilities may require us to devote additional resources and management time to manufacturing operations and may delay our product development timelines.***

We have a GMP manufacturing facility located in Houston, Texas that includes process, analytical and bioanalytical development labs with experienced teams. NGN-401 was manufactured at our Houston facility and clinical-grade product is available for dosing in the Phase 1/2 clinical trial of NGN-401 that is currently enrolling patients. However, we will need to conduct additional NGN-401 manufacturing campaigns to generate additional clinical supply, as well as supply for our preclinical studies for our discovery programs, and we may not be able to satisfy such supply through production at our own facility and may need to outsource some or all of our production work.

Other risks relating to the manufacture of biologics and drug products include: production interruptions, delays in quality/release testing, equipment malfunctions, facility contamination, labor problems, natural disasters, disruption in utility services, terrorist activities, war, cases of force majeure, weather-related events, acts of god (such as public health crises) or other events beyond our control and, in each case, could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

***Any contamination or interruption in our manufacturing process, shortages of raw materials or failure of our suppliers to deliver necessary components could result in delays in our clinical development or marketing schedules.***

Given the nature of gene therapy manufacturing, there is a risk of contamination. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage. Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects.

***We may not be able to successfully manufacture our product candidates in sufficient quality and quantity, which would delay or prevent us from developing our product candidates and commercializing resulting approved products, if any.***

To date, we have manufactured NGN-401 in quantities and quality adequate for preclinical, toxicology and clinical studies. In order to conduct clinical trials for a product candidate and for commercialization of the resulting product if that product candidate is approved for sale, we will need to manufacture product candidates in additional cGMP campaigns or in larger batch sizes. We may not be able to successfully repeat or increase the manufacturing capacity for any of our product candidates in a timely or cost-effective manner or at all. Significant changes or scale-up of manufacturing may require additional validation studies, which are costly and which regulatory authorities must review and approve. In addition, quality issues may arise during those changes or scale-up activities. If we are unable to successfully manufacture any of our product candidates in sufficient quality and quantity, the development of that product candidate and regulatory approval or commercial launch for any resulting products may be delayed or there may be a shortage in supply, which could significantly harm our business.

***Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.***

As product candidates proceed through preclinical studies to late-stage clinical trials towards potential approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are altered along the way in an effort to optimize processes and product characteristics. Such changes carry the risk that they will not achieve our intended objectives. Any such changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the materials manufactured using altered processes. Such changes may also require additional testing, FDA notification or approval from the FDA or foreign regulatory agencies. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commence sales and generate revenue. In addition, we may be required to make significant changes to our upstream and downstream processes across our pipeline, which could delay the development of our future product candidates.

## Risks Related to Our Reliance on Third Parties

***We have a number of academic collaborations, and currently rely on our collaboration with the University of Edinburgh for certain aspects of our preclinical research and development programs, including working in collaboration to discover and preclinically develop potential product candidates for our near-term future pipeline. Failure or delay of the University of Edinburgh or any other collaborator to fulfil all or part of its obligations under our agreements, a breakdown in collaboration between the parties or a complete or partial loss of the relationship would materially harm our business.***

Our discovery engine is supplemented by academic collaborations to expand our platform, which we rely upon to advance discovery and development of product candidates. For example, our collaboration with the University of Edinburgh is critical to our business. In December 2020, we entered into a Master Collaboration Agreement (the “MCA”) with the University of Edinburgh, which we rely on to conduct certain aspects of the preclinical development of our pipeline candidates, including NGN-401 and all of our early-stage pipeline product candidates. Further, in March 2022, we entered into an exclusive license agreement with the University of Edinburgh for, with respect to certain University of Edinburgh-owned technology, a worldwide, exclusive, sublicensable license to develop, have developed, use, manufacture, have manufactured, supply, have supplied, sell, have sold, offer for sale, commercialize, import, export, register, reproduce, dispose of or otherwise exploit any products, processes, components, services and/or technologies incorporating the technology for the prevention or treatment of disease or medical or genetic conditions in humans. We also currently rely on the University of Edinburgh for portions of preclinical research capabilities under the direction of Dr. Stuart Cobb, Professor in Translational Neuroscience at the University of Edinburgh and our Chief Scientific Officer. Pursuant to the MCA, we and the University of Edinburgh agreed to collaborate on certain research and development projects (the “Projects”), and we agreed to provide funding for such Projects. In exchange for such funding, the University of Edinburgh grants us an option to exclusively license any intellectual property arising from such Projects. Either party has the right in certain circumstances to terminate the collaboration pursuant to the terms of the MCA. If the MCA is not renewed or is terminated, our pipeline of product candidates would be significantly adversely affected, and our business would be materially harmed.

Following an amendment to the MCA in November 2023, the term of the research funding portion of the MCA, under which we have the ability to acquire exclusive rights to additional technology and gene therapy products, now expires in December 2026. If we need to extend the term of this provision beyond that date, we will need to negotiate an additional extension with the University of Edinburgh, and we may not be able to agree on such an extension on terms that are acceptable to us, or at all. We may have disagreements with the University of Edinburgh with respect to the interpretation of the MCA, use of resources or otherwise that could cause our relationship to deteriorate. As a result, the University of Edinburgh may reduce focus on, and resources allocated to, our programs, potentially delaying or terminating our ability to advance product candidates through preclinical studies. Additionally, if Dr. Cobb were to leave the University of Edinburgh or to otherwise no longer be meaningfully involved with us, our preclinical research and development capabilities may be substantially reduced.

Further, under the MCA, the University of Edinburgh is primarily responsible for prosecuting and maintaining our licensed intellectual property, and it may fail to properly prosecute, maintain or defend such intellectual property. In such event, if we are unable to otherwise maintain or defend such intellectual property, we could face the potential invalidation of the intellectual property or be subjected to litigation or arbitration, any of which would be time-consuming and expensive. To enforce the licensed intellectual property rights under the MCA, we will need to coordinate with the University of Edinburgh, which could slow down or hamper our ability to enforce our licensed intellectual property rights. If this happens, we could face increased competition that could materially and adversely affect our business. For a further description of the MCA, see “*Management’s Discussion and Analysis of Financial Condition and Results of Operations—License and Collaboration Agreements.*”

We also currently have or may in the future engage in other academic collaborations to supplement our internal discovery and product development program. While these academic institutions have contractual obligations to us, they are independent entities and are not under our control or the control of our officers or directors. Our research and licensing agreements with academic collaborators generally provide academic collaborators with license maintenance fees, development and regulatory milestone payments, royalties on net sales of products and a portion



of sublicense income that we receive. Upon the scheduled expiration of any academic collaboration, we may not be able to renew the related agreement, or any renewal could be on terms less favorable to us than those contained in the existing agreement. Furthermore, either we or the academic institution generally may terminate the sponsored research agreement for convenience following a specified notice period. If any of these academic institutions decides to not renew or to terminate the related agreement or decides to devote fewer resources to such activities, our discovery efforts would be diminished, while our royalty obligations, if any, would continue unmodified.

***We currently rely, and intend in the future to rely, on third parties to conduct a significant portion of our preclinical studies and existing clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.***

We have engaged CROs or other third parties to conduct preclinical and IND enabling studies and our clinical trials, including our Phase 1/2 clinical trial of NGN-401.

We expect to continue to rely on third parties, including CROs, medical institutions and clinical investigators, to conduct those clinical trials. Any of these third parties may terminate their engagements with us, some in the event of an uncured material breach and some at any time for convenience. If any of our relationships with these third parties terminate, we may not be able to timely enter into arrangements with alternative third parties or do so on commercially reasonable terms, if at all. Switching or adding CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business and financial condition.

In addition, any third parties conducting our clinical trials will not be our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not such third parties devote sufficient time and resources to our clinical programs. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated, we may incur additional and unexpected costs related to such failures, and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. Consequently, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase substantially and our ability to generate revenue could be delayed significantly.

Further, while our reliance on these third parties for research and development activities will reduce our control over these activities, we will not be relieved of our responsibilities for ensuring that each of our studies and trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with GCPs for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. If we or any of our CROs or other third parties, including trial sites, fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with GCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP conditions. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may

be required to report some of these relationships to the FDA. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of NGN-401 or any other product candidates.

We currently store drug product for clinical trial sites in the United States, and currently rely on and expect in the future to rely on third parties to distribute product supplies for our clinical trials, as well as to store and distribute supply for clinical trial sites outside of the United States. Any performance failure on the part of us or our distributors could result in an unexpected increase in costs to us, delay clinical development or marketing approval of our product candidates or commercialization of our products, if approved, producing additional losses and depriving us of potential revenue.

### **Risks Related to Our Business and Operations**

***In order to successfully implement our plans and strategies, we will need to grow the size of our organization and we may experience difficulties in managing this growth.***

Over time, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of preclinical and clinical biological product development, technical operations, clinical operations, regulatory affairs, manufacturing and, potentially, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial personnel and systems, expand our facilities and recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team working together in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

***We are highly dependent on our key personnel and anticipate hiring new key personnel. If we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.***

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our managerial, scientific and medical personnel, including our Founder and Chief Executive Officer, President and Chief Financial Officer, and Chief Scientific Officer, as well as other key members of our leadership team. Our executive officers may terminate their employment with us at any time. We do not maintain “key person” insurance for any of our executives or other employees. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key personnel may be difficult and may take an extended period of time. Failure to attract and retain qualified personnel could materially and adversely affect our business, financial condition and results of operations. We could in the future have difficulty attracting and retaining experienced personnel and may be required to expend significant financial resources on our employee recruitment and retention efforts.

***Our future growth may depend, in part, on our ability to operate in foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.***

Our future growth may depend, in part, on our ability to develop and commercialize NGN-401 or other product candidates in foreign markets for which we may rely on collaborations with third parties. We are not permitted to market or promote any product candidates before we receive regulatory approval from the applicable foreign regulatory authority, and may never receive such regulatory approval for any product candidates. To obtain separate regulatory approval in many other countries, we must comply with numerous and varying regulatory requirements of such countries regarding safety and efficacy and governing, among other things, clinical trials and commercial sales,

pricing and distribution of NGN-401 or other product candidates, and we cannot predict success in these jurisdictions. If we fail to comply with the regulatory requirements in international markets or to receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of NGN-401 or other product candidates will be harmed and our business will be adversely affected. Moreover, even if we obtain approval of NGN-401 or other product candidates and ultimately commercialize such product candidates in foreign markets, we would be subject to the risks and uncertainties of operating in such foreign markets, including the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements and reduced protection of intellectual property rights in some foreign countries.

***Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, CDMOs, suppliers and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.***

We are exposed to the risk that our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, CDMOs, suppliers and vendors acting for or on our behalf may engage in misconduct or other improper activities. It is not always possible to identify and deter misconduct by these parties and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations.

***Our internal computer systems have suffered a security breach and in the future our systems, or those of any of our CROs, manufacturers, other contractors, third party service providers or consultants or potential future collaborators, may fail or suffer additional security or data privacy breaches or other unauthorized or improper access to, use of, or destruction of our proprietary or confidential data, employee data or personal data, which could result in additional costs, loss of revenue, significant liabilities, harm to our brand and material disruption of our operations.***

Despite the implementation of security measures in an effort to protect systems that store our information, given the size and complexity of such systems and the increasing amounts of information maintained on our internal information technology systems and those of our third-party CROs, other contractors (including sites performing our clinical trials), third-party service providers and supply chain companies, consultants and other partners, these systems are potentially vulnerable to breakdown or other damage or interruption from service interruptions, system malfunction, natural disasters, terrorism, war, and telecommunication and electrical failures, as well as security breaches from inadvertent or intentional actions by our employees, contractors, consultants, business partners and/or other third parties, or from cyber-attacks by malicious third parties, which may compromise our system infrastructure or lead to the loss, destruction, alteration or dissemination of, or damage to, our data. From time to time, we are subject to business email compromise attack attempts. In August 2023, we discovered a business email compromise attack that resulted in the misappropriation of approximately \$0.9 million. While we have implemented remedial measures in response to this incident and recovered \$0.8 million of those losses through insurance claims, we cannot guarantee that such measures will prevent additional related, as well as unrelated incidents, or that we will be able to defend against or successfully remediate any such attacks that may occur in the future. If a material system failure, accident or security breach were to occur and cause interruptions in our operations or the operations of third-party collaborators, service providers, contractors and consultants, it could result in a material disruption of our development programs and significant reputational, financial, legal, regulatory, business or operational harm.

Further, since we sponsor clinical trials, any breach that compromises patient data and identities causing a breach of privacy could have significant adverse consequences on our business. For example, the loss of clinical trial data from completed or future clinical trials could affect trust in us, negatively impacting our ability to recruit for future clinical trials, result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss, destruction, unavailability, alteration or dissemination of, or damage to, our data or applications, or inappropriate disclosure of confidential proprietary information, or for it to be believed or reported that any of these occurred, we could incur liability and reputational damage and the development and commercialization of NGN-401 or other product candidates could be delayed.

As our employees work remotely and use network connections, computers, and devices outside of our premises or network, including working at home, while in transit and in public locations, there are risks to our information technology systems and data. Additionally, business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies.

While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We may be unable in the future to detect vulnerabilities in our information technology systems because such threats and techniques change frequently, are often sophisticated in nature, and may not be detected until after a security incident has occurred. Further, we may experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities. Applicable data privacy and security obligations may require us to notify relevant stakeholders, patients or other individuals, regulators or, in certain circumstances, the media of security incidents. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences, including damage to our reputation.

We rely on third-party service providers and technologies to operate critical business systems, including to process sensitive information in a variety of contexts. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If our third-party service providers experience a security incident or other interruption, we could experience adverse consequences as a result. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our monetary, reputational and other damages, or we may be unable to recover such award. In addition, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third-party partners' supply chains have not been and will not be compromised.

If we (or a third party upon whom we rely) experiences a security incident or is perceived to have experienced a security incident, we may experience adverse consequences, such as government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing personal information (including sensitive data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; interruptions in our operations (including availability of data); increased investigation and compliance costs; financial loss; and other similar harms. Security incidents and attendant consequences may cause our stakeholders (including investors and potential customers) to stop supporting our business, deter new customers from our products, deter patients from participating in clinical trials and negatively impact our ability to grow and operate our business.

Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices or from disruptions in, or failure or security breach of, our systems or third-party systems where information important to our business operations or commercial development is stored, or that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims.

***We are subject to stringent and changing laws, regulations and standards, and contractual obligations relating to privacy, data protection, and data security. The actual or perceived failure to comply with such obligations could lead to government enforcement actions (which could include civil or criminal penalties), fines and sanctions, private litigation, injunctive restrictions on data processing and/or adverse publicity and could negatively affect our operating results and business.***

We, and third parties with whom we work, are or may become subject to numerous domestic and foreign laws, regulations, and standards relating to privacy, data protection, and data security, the scope of which are changing, subject to differing applications and interpretations, and may be inconsistent among countries, or conflict with other rules. In addition, there is proposed legislation in the U.S. Congress that could restrict working with certain biotech providers who are deemed "companies of concern" which may impact the ability of certain third parties with whom

we work to meet their performance obligations. We are or may become subject to the terms of contractual obligations related to privacy, data protection, and data security. Our obligations may also change or expand as our business grows. The actual or perceived failure by us or third parties related to us to comply with such laws, regulations and obligations could increase our compliance and operational costs, expose us to regulatory scrutiny, actions, fines and penalties, result in reputational harm, lead to a loss of customers, result in litigation and liability, subject us to injunctive restrictions on data processing, adversely impact our ability to appropriately manage third parties with whom we work and otherwise cause a material adverse effect on our business, financial condition, and results of operations. See “*Business—Government Regulation—Data Privacy and Security*” and “*—Other Regulatory Matters*” in our Annual Report on Form 10-K for a more detailed description of the laws that may affect our ability to operate.

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations may involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or commercialization efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

***Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.***

As of December 31, 2023, we had net operating loss carryforwards for federal and state income tax purposes of \$277.9 million and \$35.1 million, respectively. The federal net operating losses will not be subject to expiration and can be carried forward indefinitely; however, they are limited to a deduction to 80% of annual taxable income. The state net operating losses begin to expire in 2038. To the extent that our taxable income exceeds any current year operating losses, we plan to use our carryforwards to offset income that would otherwise be taxable. Also, for state income tax purposes, the extent to which states will conform to the federal laws is uncertain and there may be periods during which the use of net operating loss carryforwards are suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. In addition, under Section 382 of the Code, changes in our ownership may limit the amount of our net operating loss carryforwards and tax credit carryforwards that could be utilized annually to offset our future taxable income, if any. This limitation would generally apply in the event of a cumulative change in ownership of more than 50% (as measured by value) among a stockholder or one or more groups of stockholders who own at least 5% of our stock within a three-year period. We have not performed an analysis to determine whether there has been an ownership change pursuant to Section 382. Any such limitation may significantly reduce our ability to utilize our net operating loss carryforwards and tax credit carryforwards before they expire. Any such limitation, whether as the result of a public offering, private placements, sales of our common stock by our existing stockholders or additional sales of our common stock by us, could have a material adverse effect on our results of operations in future years.

***We may be subject to adverse legislative or regulatory tax changes that could negatively impact our financial condition.***

The rules dealing with U.S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or our stockholders. We assess the impact of various tax reform proposals and modifications to existing tax treaties in all jurisdictions where we have operations to determine the potential effect on our business and any assumptions we have made about our future taxable income. We cannot predict whether any specific proposals will be enacted, the terms of any such proposals or what effect, if any, such proposals would have on our business if they were to be enacted. For example, the United States recently enacted the Inflation Reduction Act of 2022, which implements, among other changes, a 1% excise tax on certain stock buybacks. In addition, beginning in 2022, the Tax Cuts and Jobs Act eliminates the currently available option to deduct research and development expenditures and requires taxpayers to amortize them

generally over five years. The U.S. Congress is considering legislation that would restore the current deductibility of research and development expenditures, however, there is no assurance that the provision will be repealed or otherwise modified. Such changes, among others, may adversely affect our effective tax rate, results of operation and general business condition.

***We may acquire businesses or products, or form strategic alliances, in the future, and may not realize the benefits of such acquisitions.***

We may acquire additional businesses or products, form strategic alliances, or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new product candidates or products resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. There is no assurance that, following any such acquisition, we will achieve the synergies expected in order to justify the transaction, which could result in a material adverse effect on our business and prospects.

***We maintain our cash at financial institutions, at times in balances that exceed federally-insured limits. The failure of financial institutions could adversely affect our ability to pay our operational expenses or make other payments.***

Our cash held in non-interest-bearing and interest-bearing accounts at financial institutions can at times exceed the Federal Deposit Insurance Corporation (“FDIC”) insurance limits. If such banking institutions were to fail, we could lose all or a portion of those amounts held in excess of such insurance limitations. For example, the FDIC took control of Silicon Valley Bank on March 10, 2023. The Federal Reserve subsequently announced that account holders would be made whole. However, the FDIC may not make all account holders whole in the event of future bank failures. In addition, even if account holders are ultimately made whole with respect to a future bank failure, account holders’ access to their accounts and assets held in their accounts may be substantially delayed. Any material loss that we may experience in the future or inability for a material time period to access our cash and cash equivalents could have an adverse effect on our ability to pay our operational expenses or make other payments, which could adversely affect our business.

***At the end of August 2023, we identified a material weakness in our internal control over financial reporting and may identify additional material weaknesses in the future that may cause us to fail to meet our reporting obligations or result in material misstatements of our financial statements. If our internal control over financial reporting or our disclosure controls and procedures are not effective, we may not be able to accurately report our financial results, prevent fraud or file our periodic reports in a timely manner, which may cause investors to lose confidence in our reported financial information and may lead to a decline in our share price.***

Our internal controls related to the cash disbursements process were not adequately designed to identify unauthorized payment requests, resulting in the identification of a material weakness. Specifically, at the end of August 2023, we discovered that we were subject to a business email compromise attack by a third party. This deficiency in our controls resulted in the diversion of payments to fraudulent bank accounts.

We determined that certain internal controls required for safeguarding our cash assets were not properly designed due to insufficient specificity regarding our policies and procedures surrounding supplier banking information changes, not identifying segregation of duties, and insufficient training on exercising professional skepticism. We therefore implemented steps to remediate this control deficiency, including increasing communication of and training around our controls relating to changes made to information, emphasizing security awareness and the importance of professional skepticism and designing a process to review supplier information changes prior to release of payments. While our management determined based on the assessment of internal control over financial reporting that as of December 31, 2023, this material weakness had been remediated, there can be no assurance that the remediation plans we implemented relating to this business email compromise attack will be successful in preventing a repeat of that attack or that we will be able to avoid potential future material weaknesses. If we are unable to successfully remediate existing or any future material weakness in our internal control over

financial reporting, or if we identify any additional material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law and applicable stock exchange listing requirements regarding timely filing of periodic reports, investors may lose confidence in our financial reporting, and our stock price may decline as a result. We also could become subject to investigations by Nasdaq, the SEC or other regulatory authorities.

### **Risks Related to Intellectual Property**

#### ***Our ability to protect our patents and other proprietary rights is uncertain, exposing us to the possible loss of competitive advantage.***

We rely and expect to continue to rely upon a combination of patents, trademarks, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates and technologies and to prevent third parties from unfairly competing with us. Our success depends in large part on our ability to obtain and maintain patent protection for platform technologies, including our EXACT transgene regulation platform, product candidates and their uses, as well as the ability to operate without infringing on or violating the proprietary rights of others. As of September 30, 2024, we license 29 patent applications, including U.S. patent applications, international patent applications under the Patent Cooperation Treaty or otherwise, and expect to continue to file patent applications in the United States and abroad related to discoveries and technologies that are important to our business. However, we may not be able to protect our intellectual property rights throughout the world and the legal systems in certain countries may not favor enforcement or protection of patents, trade secrets and other intellectual property. Filing, prosecuting and defending patents on product candidates worldwide would be prohibitively expensive and our intellectual property rights in some foreign jurisdictions may be less extensive than those in the United States. As such, we do not have patents in all countries or all major markets and may not be able to obtain patents in all jurisdictions even if we apply for them. Competitors may operate in countries where we do not have patent protection and could then freely use our technologies and discoveries in such countries to the extent such technologies and discoveries are publicly known or disclosed in countries where patent protection has not been requested. In addition, competitors may be able to design around our patents to create technologies that directly compete with ours without infringing our intellectual property.

Our intellectual property portfolio is at an early stage. As of September 30, 2024, we do not own or in-license any issued patents. Our pending and future patent applications may not result in patents being issued. Any issued patents may not afford sufficient protection of our product candidates or their intended uses against competitors, nor can there be any assurance that the patents issued will not be infringed, designed around, invalidated by third parties, or effectively prevent others from commercializing competitive technologies, products or product candidates. Even if these patents are granted, they may be difficult to enforce. Further, any issued patents that may be licensed or owned covering our product candidates could be narrowed or found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad, including the United States Patent and Trademark Office (“USPTO”). Further, if we encounter delays in any clinical trials or delays in obtaining regulatory approval, the period of time during which we could market product candidates under patent protection would be reduced. Thus, the patents that we may own or license may not afford any meaningful competitive advantage.

In addition to seeking patents for some of our technology and product candidates, we may also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. Any disclosure, either intentional or unintentional, by our employees, the employees of third parties with whom we share facilities or third-party consultants and vendors that we engage to perform researches, clinical trials or manufacturing activities, or misappropriation by third parties (such as through a cybersecurity breach) of our trade secrets or proprietary information could enable competitors to duplicate or surpass our technological achievements, thus eroding our competitive position in the market. In order to protect our proprietary technology and processes, we rely in part on confidentiality agreements with collaborators, employees, consultants, outside scientific collaborators and sponsored researchers and other advisors. These agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. We may need to share our proprietary information, including trade secrets, with future business partners, collaborators, contractors and others located in countries at heightened risk of theft of

trade secrets, including through direct intrusion by private parties or foreign actors and those affiliated with or controlled by state actors. In addition, while we undertake efforts to protect our trade secrets and other confidential information from disclosure, others may independently discover trade secrets and proprietary information, and in such cases, we may not be able to assert any trade secret rights against such party. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

Lastly, if our trademarks and trade names are not registered or adequately protected, then we may not be able to build name recognition in markets of interest and our business may be adversely affected.

***We may not be successful in obtaining or maintaining necessary rights to product candidates through acquisitions and in-licenses.***

Because our development programs require and may in the future require the use of proprietary rights held by third parties, the growth of our business may depend in part on our ability to acquire, in-license, or use these third-party proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary for product candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on investment or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant product candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

While we will normally seek to obtain the right to control prosecution, maintenance and enforcement of the patents relating to a product candidate, there may be times when the filing and prosecution activities for patents and patent applications relating to a product candidate are controlled by future licensors or collaboration partners. For example, we currently license several patent families from the University of Edinburgh covering the EXACT transgene regulation platform, as well as the NGN-401 product candidate and its uses. If any of such licensors or collaboration partners fail to prosecute, maintain and enforce such patents and patent applications in a manner consistent with the best interests of our business, including by payment of all applicable fees for patents covering a product candidate, we could lose rights to the intellectual property or exclusivity with respect to those rights, our ability to develop and commercialize such candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products. In addition, even where we have the right to control patent prosecution of patents and patent applications which may be licensed to and from third parties, we may still be adversely affected or prejudiced by actions or inactions of licensees, future licensors and their counsel that took place prior to the date upon which we assumed control over patent prosecution.

Our future licensors may rely on third-party consultants or collaborators or on funds from third parties such that future licensors are not the sole and exclusive owners of the patents we in-license. If other third parties have ownership rights to future in-licensed patents, they may be able to license such patents to our competitors, and the competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

It is possible that we may be unable to obtain licenses at a reasonable cost or on reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing the same, or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business, financial condition, results of operations, and prospects significantly. We cannot provide any assurances that third-party



patents do not exist which might be enforced against our current technology or manufacturing methods, our product candidates, or future methods or product candidates, resulting in either an injunction prohibiting manufacture or future sales, or, with respect to future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

Disputes may arise between us and our future licensors regarding intellectual property subject to a license agreement, including: the scope of rights granted under the license agreement and other interpretation-related issues; whether and to what extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement; our right to sublicense patents and other rights to third parties; our right to transfer or assign the license; the inventorship and ownership of inventions and know-how resulting from the joint creations or use of intellectual property by future licensors and us and/or our partners; and the priority date of an invention of patented technology.

***Certain of our current product candidates and research programs are licensed from or based upon licenses from a third party and are field limited to certain indications. If these license agreements are terminated or interpreted to narrow our rights, our ability to advance our current product candidates or develop new product candidates based on these technologies will be materially adversely affected.***

We depend on, and will continue to depend on, our current licenses with the University of Edinburgh, Virovek, Inc. (“Virovek”), Sigma-Aldrich Co. LLC (“Sigma”), and Leland Stanford Junior University (“Stanford”), and on licenses and sublicenses from other third parties, as well as potentially on other strategic relationships with third parties, for the research, development, manufacturing and commercialization of our current product candidates. If any of our licenses or relationships or any in-licenses on which our licenses are based are terminated or breached, we may:

- lose our rights to develop and market our current product candidates;
- lose patent or trade secret protection for our current product candidates;
- experience significant delays in the development or commercialization of our current product candidates;
- not be able to obtain any other licenses on acceptable terms, if at all; or
- incur liability for damages.

Additionally, even if not terminated or breached, our intellectual property licenses or sublicenses may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations.

If we experience any of the foregoing, it could have a materially adverse effect on our business and could force us to cease operations.

***If we fail to comply with our obligations in any agreements under which we may license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.***

We are party to license agreements with the University of Edinburgh, Virovek, Sigma and Stanford and may from time to time in the future be party to other license and collaboration agreements with third parties to advance our research or allow commercialization of current or future product candidates. Such agreements may impose numerous obligations, such as development, diligence, payment, commercialization, funding, milestone, royalty, sublicensing, insurance, patent prosecution, enforcement and other obligations on us and may require us to meet development timelines, or to exercise commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the licenses. Despite our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technologies covered by these license agreements.

If these licenses are terminated for any reason, or if the underlying patents fail to provide the intended exclusivity, we could lose significant rights and our ability to commercialize our current or future product candidates may be harmed, and competitors or other third parties would have the freedom to seek regulatory approval of, and to market, products identical to ours and we may be required to cease our development and commercialization of certain of our current or future product candidates. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Disputes may also arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe, misappropriate or otherwise violate intellectual property rights of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to the development and commercialization of our current or future product candidates, and what activities satisfy those diligence obligations;
- the priority of invention of any patented technology; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our current or future licensors and by us and our other partners.

In addition, the agreements under which we may license intellectual property or technology from third parties are likely to be complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we may license prevent or impair our ability to maintain future licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected current or future product candidates, which could have a material adverse effect on our business, financial conditions, results of operations and prospects.

***We may be subject to patent infringement claims or may need to file claims to protect our intellectual property, which could result in substantial costs, liability and diversion of resources, and prevent or delay us from commercializing potential products.***

Because the intellectual property landscape in the biotechnology industry is rapidly evolving and interdisciplinary, it is difficult to conclusively assess our freedom to operate and guarantee that we can operate without infringing on or violating third party rights. If certain of our product candidates are ultimately granted regulatory approval, patent rights held by third parties, if found to be valid and enforceable, could be alleged to render one or more of such product candidates infringing. We cannot be certain that patents owned or licensed by us will not be challenged by others in the course of litigation. If a third party successfully brings a claim against us, we may be required to pay substantial damages, be forced to abandon any affected product candidate and/or seek a license from the patent holder. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our business.

Competitors may infringe or otherwise violate our patents, trademarks, copyrights or other intellectual property. To counter infringement or other violations, we may be required to file claims, which can be expensive and time-consuming. Any such claims could provoke these parties to assert counterclaims against us, including claims alleging that our intellectual property, methods or products infringes their patents or other intellectual property rights. In addition, in a patent infringement proceeding, a court or administrative body may decide that one or more

of the patents we assert is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or refuse to prevent the other party from using the technology at issue on the grounds that our patents do not cover the technology. Similarly, if we assert trademark infringement claims, a court or administrative body may determine that the marks asserted are invalid or unenforceable or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In such a case, we could ultimately be forced to cease use of such marks. In any intellectual property litigation, even if we are successful, any award of monetary damages or other remedy received may not be commercially valuable.

Further, we may be required to protect our patents through procedures created to attack the validity of a patent at the USPTO. An adverse determination in any such submission or proceeding could reduce the scope or enforceability of, or invalidate, our patent rights, which could adversely affect our competitive position. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action.

If we are required to defend intellectual property actions brought by third parties, or if we sue to protect our own intellectual property rights or otherwise to protect our proprietary information and to prevent its disclosure, or if we are involved in other litigation, whether as a plaintiff or defendant, and whether or not successful, we may incur substantial legal expenses and the attention of our management and key personnel may be diverted from business operations. Further, some of our competitors may be able to sustain the costs of complex intellectual property litigation more effectively than we can because they have substantially greater resources.

In addition, if our product candidates are found to infringe the intellectual property rights of third parties, these third parties may assert infringement claims against our future licensees and other parties with whom we have business relationships and we may be required to indemnify those parties for any damages they suffer as a result of these claims, which may require us to initiate or defend protracted and costly litigation on behalf of licensees and other parties regardless of the merits of such claims. If any of these claims succeed, we may be forced to pay damages on behalf of those parties or may be required to obtain licenses for the products they use, and may not be able to obtain such licenses on terms acceptable to us, if at all.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or other legal proceedings relating to our intellectual property rights, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation or other proceedings.

***We may be subject to claims that we have wrongfully hired an employee from a competitor or that employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.***

As is common in the biotechnology industry, in addition to employees, we engage consultants to assist in the development of our product candidates. Many of these consultants, and many of our employees, were or may have been previously employed at, or may have previously provided or may be currently providing consulting services to, other biotechnology or pharmaceutical companies including our competitors or potential competitors. We could in the future be subject to claims that we or our employees or consultants working on our behalf have inadvertently or otherwise used or disclosed alleged trade secrets or other confidential information of former employers or competitors. Although we try to ensure that our employees and consultants do not use the intellectual property, proprietary information, know-how or trade secrets of others in their work for us, we may become subject to claims that we caused an employee to breach the terms of his or her non-competition or non-solicitation agreement, or that we or these individuals have, inadvertently or otherwise, used or disclosed the alleged trade secrets or other proprietary information of a former employer or competitor.

We may litigate to defend ourselves against these claims, and even if we are successful, litigation could result in substantial costs and could be a distraction to management. If our defenses to these claims fail, in addition to requiring us to pay monetary damages, a court could prohibit us from using technologies or features that are essential to our product candidates, if such technologies or features are found to incorporate or be derived from the

trade secrets or other proprietary information of the former employers. Moreover, any such litigation or the threat thereof may adversely affect our reputation, our ability to form strategic alliances or sublicense our rights to collaborators, engage with scientific advisors or hire employees or consultants, each of which would have an adverse effect on our business, operations and financial condition.

***Changes to patent laws in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.***

Changes in either the patent laws or interpretation of patent laws in the United States, including patent reform legislation such as the Leahy-Smith America Invents Act (the “Leahy-Smith Act”), could increase the uncertainties and costs surrounding the prosecution of our owned and any future in-licensed patent applications and the maintenance, enforcement or defense of our owned and any future in-licensed issued patents. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These changes include provisions that affect the way patent applications are prosecuted, redefine prior art, provide more efficient and cost-effective avenues for competitors to challenge the validity of patents, and enable third-party submission of prior art to the USPTO during patent prosecution along with additional procedures to attack the validity of a patent at USPTO-administered post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings. Assuming that other requirements for patentability are met, prior to March 16, 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 16, 2013, under the Leahy-Smith Act, the United States transitioned to a first-to-file system in which, assuming that the other statutory requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. As such, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, our operations and prospects.

In addition, the patent positions of companies in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. U.S. Supreme Court and U.S. Court of Appeals for the Federal Circuit rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations, including in the antibody arts. This combination of events has created uncertainty with respect to the validity and enforceability of patents once obtained. Depending on future actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our patent rights and our ability to protect, defend and enforce our patent rights in the future.

Geopolitical actions in the United States and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of patent applications and the maintenance, enforcement or defense of issued patents. Accordingly, our competitive position may be impaired, and our business, financial condition, operations and prospects may be adversely affected.

In addition, a European Unified Patent Court (“UPC”) came into force in June 2023. The UPC is a common patent court to hear patent infringement and revocation proceedings effective for member states of the European Union. This could enable third parties to seek revocation of a European patent in a single proceeding at the UPC rather than through multiple proceedings in each of the jurisdictions in which the European patent is validated. We currently have three pending European applications, and if we obtain such patents and applications in the future, any such revocation and loss of patent protection could have a material adverse impact on our business and our ability to commercialize or license our technology and products. Moreover, the controlling laws and regulations of the UPC will develop over time, and may adversely affect our ability to enforce or defend the validity of any European patents obtained. We may decide to opt out from the UPC for any future European patent applications that we may file and any patents we may obtain. If certain formalities and requirements are not met, however, such European patents and patent applications could be challenged for non-compliance and brought under the jurisdiction of the UPC. We cannot be certain that future European patents and patent applications will avoid falling under the jurisdiction of the UPC, if we decide to opt out of the UPC.

***Obtaining and maintaining patent protection depends on compliance with various procedural, document submissions, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.***

Periodic maintenance fees, renewal fees, annuities fees and various other governmental fees on patents and/or patent applications are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent and/or patent application. The USPTO and various foreign governmental patent agencies also require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

***We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop and market our products.***

We cannot guarantee that any of our patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction. The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect. For example, we may incorrectly determine that our products are not covered by a third-party patent or may incorrectly predict whether a third party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our products.

In addition, because some patent applications in the United States may be maintained in secrecy until the patents are issued, patent applications in the United States and many foreign jurisdictions are typically not published until 18 months after filing, and publications in the scientific literature often lag behind actual discoveries, we cannot be certain that others have not filed patent applications for technology covered by our pending applications or any future issued patents, or that we were the first to invent the technology. Our competitors may have filed, and may in the future file, patent applications covering our products or technology similar to ours. Any such patent application may have priority over our patent applications or patents, which could require us to obtain rights to issued patents covering such technologies.

***We may become subject to claims challenging the inventorship or ownership of our patents and other intellectual property.***

We may be subject to claims that former employees, collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing our product candidates or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are

successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Our current or future licensors may have relied on third-party consultants or collaborators or on funds from third parties, such as the U.S. government or academic institutions, such that our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights or other rights to our in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, operations, and prospects.

***Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.***

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and future licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

***Some intellectual property that we have in-licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as “march-in” rights, certain reporting requirements and a preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.***

Certain of the intellectual property rights we have licensed are generated through the use of U.S. government funding and are therefore subject to certain federal regulations. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980 (the “Bayh-Dole Act”) and implementing regulations. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require our or our licensors’ to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as “march-in rights”). The U.S. government also has the right to take title to these inventions if we fail, or the applicable licensor, fails to disclose the invention to the government and fails to file an application to register the intellectual property within specified time limits. These time limits have recently been changed by regulation, and may change in the future. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

## Risks Related to Government Regulation

***The regulatory approval processes of the FDA and other comparable foreign regulatory authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for our product candidates, or if we determine that we are not willing or able to complete the regulatory approval process given the resources required to do so, we will not be able to commercialize, or will be delayed in commercializing, such product candidates, and our ability to generate revenue will be materially impaired.***

The process of obtaining regulatory approvals, both in the United States and abroad, is unpredictable, expensive and typically takes many years following commencement of clinical trials, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. While our product candidate NGN-401 for Rett syndrome has been accepted into the FDA's START program and the RMAT program, which we expect together will allow us to have access to more frequent advice from FDA staff, intensive guidance on efficient drug development and eligibility for Accelerated Approval pathway and Priority Review, participation in these programs is not a guarantee that our approval process with the FDA will be faster or that we will ultimately achieve approval of a pivotal trial design or approval of NGN-401 as an accepted therapy for Rett syndrome. We cannot commercialize product candidates in the United States without first obtaining regulatory approval from the FDA. In addition, we may determine that the resources required to complete the regulatory approval process are in excess of what we are able or willing to expend on a particular program. For example, in November 2024, we announced that the Company does not expect to move forward with the NGN-101 CLN5 Batten disease gene therapy program. Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. We are currently evaluating options for the program.

Similarly, we cannot commercialize product candidates outside of the United States without obtaining regulatory approval from comparable foreign regulatory authorities. Before obtaining regulatory approvals for the commercial sale of our product candidates, including our most advanced product candidate, NGN-401, we must demonstrate through lengthy, complex and expensive preclinical and clinical trials that such product candidates are safe, pure and effective or potent for each targeted indication.

Securing regulatory approval also requires the submission of information about the biological product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Further, a product candidate may not be effective or potent, may be only moderately effective or potent or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude its obtaining marketing approval. The FDA and comparable foreign regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other data. A product candidate could be delayed in receiving, or fail to receive, regulatory approval for many reasons, including: the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials; we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe, pure, and effective or potent for its proposed indication; the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval; serious and unexpected product-related side effects may be experienced by participants in our clinical trials or by individuals using drugs or biological products similar to a product candidate; we may be unable to demonstrate that a candidate's clinical and other benefits outweigh its safety risks; the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials; the data collected from clinical trials of a product candidate may not be acceptable or sufficient to support the submission of a BLA or other submission or to obtain regulatory approval in the United States or elsewhere, and we may be required to conduct additional clinical trials; the FDA or the applicable foreign regulatory authority may disagree regarding the formulation, labeling and/or the specifications of a product candidate; the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and

the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Of the large number of products in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in us failing to obtain regulatory approval to market NGN-401 or other product candidates, which would significantly harm our business, results of operations and prospects.

If we were to obtain approval, regulatory authorities may approve any such product candidate for fewer or more limited indications than we request, including failing to approve the most commercially promising indications, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for a product candidate, we will not be able to commercialize, or will be delayed in commercializing, such product candidate and our ability to generate revenue may be materially impaired.

***Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict the time and cost of obtaining regulatory approval, if received at all, for any product candidates we may develop.***

The regulatory requirements that will govern any novel gene therapy product candidates we develop are not entirely clear and are subject to change. Within the broader genetic medicine field, very few therapeutic products have received marketing authorization from the FDA or the EMA. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products and cell therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial overlap in those responsible for review and regulation of existing gene therapy products and cell therapy products. For example, in the United States, the FDA has established the Office of Therapeutic Products within its Center for Biologics Evaluation and Research (“CBER”), as part of its reorganization of the Office of Tissues and Advanced Therapies, to consolidate the review of gene therapy and related products. In addition, the Cellular, Tissue and Gene Therapies Advisory Committee advises CBER on its review.

Our product candidates will need to meet safety, purity and efficacy or potency standards applicable to any new biologic under the regulatory framework administered by the FDA. In addition to FDA oversight and oversight by IRBs under guidelines promulgated by the National Institutes of Health (“NIH”) gene therapy clinical trials are also subject to review and oversight by an institutional biosafety committee (“IBC”), a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment. While the NIH guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH guidelines voluntarily follow them. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation.

The same applies in the European Union. The EMA’s Committee for Advanced Therapies (“CAT”) is responsible for assessing the quality, safety, and efficacy of advanced-therapy medicinal products. Advanced-therapy medicinal products include gene therapy medicines, somatic-cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any gene therapy product candidate we may develop, but that remains uncertain at this point.



Adverse developments in preclinical studies or clinical trials conducted by others in the field of gene therapy and gene regulation products may cause the FDA, the EMA, and other regulatory authorities to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing gene regulation technologies, either of which could harm our business. In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory authorities and the criteria these regulators use to determine the safety, purity and efficacy or potency of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. Because of this complexity, even though our product candidate NGN-401 for Rett syndrome has been accepted into the FDA's START program and the RMAT program, which we expect together will allow us to have access to more frequent advice from FDA staff, intensive guidance on efficient drug development and eligibility for an Accelerated Approval pathway and Priority Review, the regulatory approval process for product candidates such as those being developed by us can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Further, as we are developing novel potential treatments for diseases in which, in some cases, there is little clinical experience with potential new endpoints and methodologies, heightened risk that the FDA, the EMA or other regulatory authorities may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. In addition, we may not be able to identify or develop appropriate animal disease models to enable or support planned clinical development. Any natural history studies that we may conduct or rely upon in our clinical development may not be accepted by the FDA, EMA or other regulatory authorities. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene regulation technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the commercialization of resulting products. Further, approvals by one regulatory agency may not be indicative of what other regulatory agencies may require for approval.

The regulatory review committees and advisory groups described above and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

***Disruptions at the FDA and other regulatory authorities could negatively affect the review of our regulatory submissions, which could negatively impact our business.***

The ability of the FDA and other regulatory authorities to review and approve regulatory submissions can be affected by a variety of factors, including understaffing, disruptions caused by government shutdowns and public health crises. Such disruptions could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

***We may not be able to meet requirements for the chemistry, manufacturing and control of our product candidates.***

In order to receive approval of our products by the FDA and comparable foreign regulatory authorities, we must show that we and our contract manufacturing partners are able to characterize, control and manufacture our biological products safely and in accordance with regulatory requirements. This includes manufacturing the drug substance, developing an acceptable formulation, performing tests to adequately characterize the formulated product, documenting a repeatable manufacturing process, and demonstrating that our biological products meet stability requirements. Meeting these chemistry, manufacturing and control ("CMC") requirements is a complex task that requires specialized expertise. If we are not able to meet the CMC requirements, we may not be successful in getting our products approved.

***We intend to deliver our product candidates via a drug delivery device that will have its own regulatory, development, supply and other risks.***

We intend to deliver our product candidates via a drug delivery device, such as a catheter or other delivery system. There may be unforeseen technical complications related to the development activities required to bring such a product to market, including primary container compatibility and/or dose volume requirements. We expect to use drug delivery devices authorized for marketing under clearances or approvals held by third parties. Our product candidates may not be approved or may be substantially delayed in receiving approval if the devices do not gain and/or maintain their own regulatory approvals or clearances. Where approval of the drug product and device is sought under a single application, the increased complexity of the review process may delay approval. In addition, some drug delivery devices are provided by single-source unaffiliated third-party companies. We may be dependent on the sustained cooperation and effort of those third-party companies both to supply the devices and, in some cases, to conduct the studies required for approval or other regulatory clearance of the devices. Even if approval is obtained, we may also be dependent on those third-party companies continuing to maintain such approvals or clearances once they have been received. Failure of third-party companies to supply the devices, to successfully complete studies on the devices in a timely manner, or to obtain or maintain required approvals or clearances of the devices could result in increased development costs, delays in or failure to obtain regulatory approval and delays in product candidates reaching the market or in gaining approval or clearance for expanded labels for new indications.

***We currently and may in the future conduct clinical trials for our product candidates at sites outside the United States, and the FDA may not accept data from trials conducted in such locations.***

We plan to conduct clinical trials outside the United States, including in Australia, the UK, Europe or other foreign jurisdictions. For example, we are currently conducting our Phase 1/2 clinical trials for NGN-401 in the United States, the UK and Australia. In cases where data from clinical trials conducted outside the United States are intended to serve as the sole basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the United States population and United States medical practice; (ii) the trials were performed by clinical investigators of recognized competence and (iii) the data may be considered valid without the need for an on-site inspection by the FDA or, if the FDA considers such an inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. Additionally, the FDA's clinical trial requirements, including sufficient size of patient populations and statistical powering, must be met. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any similar foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA or any similar foreign regulatory authority does not accept such data, it would result in the need for additional trials, which would be costly and time-consuming and delay aspects of our business plan, and which may result in our product candidates not receiving approval or clearance for commercialization in the applicable jurisdiction. Even if the FDA accepts such data, it could require us to modify our planned clinical trials to receive clearance to initiate such trials in the United States or to continue such trials once initiated.

Other risks inherent in conducting international clinical trials include: foreign regulatory requirements, differences in healthcare services, and differences in cultural customs that could restrict or limit our ability to conduct our clinical trials; administrative burdens of conducting clinical trials under multiple sets of foreign regulations; foreign exchange fluctuations; diminished protection of intellectual property in some countries; and political and economic risks relevant to foreign countries.

***Our product candidates for which we intend to seek approval as biologics may face competition sooner than anticipated.***

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 ("BPCIA"), which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a highly similar or "biosimilar" product may not be submitted to the FDA until four years following the date that the reference product was first

approved by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first approved. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product.

Our investigational biological products, if approved, could be considered reference products entitled to 12-year exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider a product candidate to be reference products for competing products, potentially creating the opportunity for competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

***Even if we receive regulatory approval of NGN-401 or other product candidates, we will be subject to extensive ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.***

Any regulatory approvals that we may receive for NGN-401 or other product candidates will require the submission of reports to regulatory authorities and surveillance to monitor the safety, purity and efficacy or potency of such product candidates, may contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, and may include burdensome post-approval study or risk management requirements. For example, the FDA may require a risk evaluation and mitigation strategy in order to approve a product candidate, which could entail requirements for a medication guide, physician training and communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or comparable foreign regulatory authorities approve a product candidate, the products and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, purity, efficacy or potency, recordkeeping, labeling, storage, approval, advertising, promotion, sale, distribution, import and export will be subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable foreign regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as on-going compliance with current cGMPs and GCPs for any clinical trials that we conduct following approval. In addition, manufacturers of drug products and their facilities are subject to continual review and periodic, unannounced inspections by the FDA and other regulatory authorities for compliance with cGMPs.

If we or a regulatory authority discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facilities where the product is manufactured, a regulatory authority may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing, requiring the addition of labeling statements, such as a "black box" warning or a contraindication, requiring creation of a medication guide outlining the risk of such side effects for distribution to patients, withdrawal or suspension of existing approvals or licenses, refusal to approve pending applications or supplements, restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials, restrictions on the manufacturing process, warning or untitled letters, civil and criminal penalties, injunctions, product seizures, detentions or import bans, voluntary or mandatory publicity requirements and imposition of restrictions on operations, including costly new manufacturing requirements. The occurrence of any event or penalty described above may inhibit our ability to commercialize NGN-401 or other product candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity.

***We may face difficulties from healthcare legislative reform measures.***

Existing regulatory policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of NGN-401 or other product candidates. We cannot predict the

likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability. See “*Business—Government Regulation—Healthcare Reform*” in our Annual Report on Form 10-K for a more detailed description of healthcare reforms measures that may prevent us from being able to generate revenue, attain profitability, or commercialize product candidates.

***Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties.***

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers may expose us to broadly-applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. See “*Business—Government Regulation—Other Healthcare Laws and Compliance Requirements*” in our Annual Report on Form 10-K for a more detailed description of the laws that may affect our ability to operate.

Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. If our operations are found to be in violation of any of these laws or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations. Further, defending against any such actions can be costly and time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

***Even if we are able to commercialize NGN-401 or other product candidates, due to unfavorable pricing regulations and/or third-party coverage and reimbursement policies, we may not be able to offer such products at competitive prices which would seriously harm our business.***

We intend to seek approval to market NGN-401 and other product candidates in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for such product candidates, we will be subject to rules and regulations in those jurisdictions. Our ability to successfully commercialize any product candidates that we may develop will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. These entities may create preferential access policies for a competitor’s product, including a branded or generic/biosimilar product, over our products in an attempt to reduce their costs, which may reduce our commercial opportunity. Additionally, if any of our product candidates are approved and we are found to have improperly promoted off-label uses of those programs, we may become subject to significant liability, which would materially adversely affect our business and financial condition. See “*Business—Government Regulation—Coverage and Reimbursement*” and “*—Regulation in the European Union*” in our Annual Report on Form 10-K for a more detailed description of the government regulations and third-party payor practices that may affect our ability to commercialize product candidates.

***We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws, and anti-money laundering laws and regulations. We can face criminal liability and other serious consequences for violations, which can harm our business.***

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, the U.S. Foreign Corrupt Practices Act of 1977, as amended, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. In addition, the U.S. Congress is currently contemplating legislation that could have the impact of limiting the ability of us and certain of our vendors to work with certain designated biotech companies from China and other nations.

Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors, and other collaborators from authorizing, promising, offering, or providing, directly or indirectly, improper payments or anything else of value to or from recipients in the public or private sector. We may engage third parties to sell products outside the United States, to conduct clinical trials, and/or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors, and other collaborators, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences.

***Governments outside the United States may impose strict price controls, which may adversely affect our revenue, if any.***

In some countries, particularly member states of the European Union, the pricing of prescription drugs is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after receipt of marketing approval for a therapeutic. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. To obtain coverage and reimbursement or pricing approvals in some countries, we or current or future collaborators of ours may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of a product to other available therapies in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If reimbursement of any product approved for marketing is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business, financial condition, results of operations or prospects could be materially and adversely affected. Brexit could lead to legal uncertainty and potentially divergent national laws and regulations, including those related to the pricing of prescription pharmaceuticals, as the UK determines which EU laws to replicate or replace. If the UK were to significantly alter its regulations affecting the pricing of prescription pharmaceuticals, we could face significant new costs.

***While we have received Fast Track designation for NGN-401 for the treatment of Rett syndrome and NGN-401 has been accepted into the FDA's START Pilot Program and RMAT program, such designations may not lead to a faster development or regulatory review or approval process.***

In 2024, the FDA began accepting applications from sponsors for the Support for Clinical Trials Advancing Rare disease Therapeutics (START) pilot program with the purpose of further accelerating the pace of development of novel drug and biological products that are intended to address an unmet medical need as a treatment for rare disease. The pilot is designed to be milestone-driven (i.e. to facilitate the progression of a development program to

pivotal clinical study stage or the pre-BLA meeting stage) where product development programs selected would benefit from enhanced communication with the FDA. The START pilot program is intended to provide a mechanism for addressing clinical development issues that otherwise would delay or prevent a promising novel drug or biological product from progressing to the pivotal clinical trial stage or pre-BLA meeting stage. Participants in the START Pilot Program will receive enhanced communications with the FDA review staff. These enhanced communications will include at a minimum an initial meeting to review features of the pilot, discuss a pathway intended to support a marketing application, and to discuss specific issues for which a sponsor requests enhanced communications with the FDA. Additional communications will include ongoing interactions via email or teleconference that take place on a scheduled and/or as needed basis as agreed upon by the sponsor information on how best to facilitate more efficient development of potentially life-saving therapies for rare diseases and help sponsors generate high-quality, actionable data to support future new drug or biologics license applications. In June 2024, we announced that our product candidate NGN-401 had been accepted into the FDA's START Pilot Program, which we expect will allow us to have access to more frequent advice from FDA staff to address product-specific development issues, possibly including clinical study design, choice of control group, patient population choices and other early development issues.

The FDA's RMAT designation program is intended to fulfill the requirement of the 21st Century Cures Act that the FDA facilitate an efficient development program for, and expedite review of, any product that meets the following criteria: (1) it qualifies as an RMAT, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions; (2) it is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and (3) preliminary clinical evidence indicates that the product has the potential to address unmet medical needs for such a disease or condition. In 2024, the FDA granted an RMAT designation for NGN-401 for the treatment of Rett syndrome. RMAT designation provides potential benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate, and eligibility for rolling review and priority review. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or may be able to rely upon data obtained from a meaningful number of sites, including through expansion to additional sites. RMAT designation does not change the standards for product approval, and there is no assurance that such designation will result in expedited review or approval or that the approved indication will not be narrower than the indication covered by the RMAT designation. Additionally, RMAT designation can be revoked if the criteria for eligibility cease to be met as clinical data emerges.

The FDA may also designate a product for Fast Track review if it is intended, whether alone or in combination with one or more other products, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For Fast Track products, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of a Fast Track product's application before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a Fast Track product may be effective. We have received Fast Track designation in the United States for NGN-401 for the treatment of Rett syndrome, and we may seek additional designations for one or more of our other product candidates that could expedite review and approval by the FDA.

Participation in the START Pilot Program, RMAT designation and the designation of a product for Fast Track review are within the discretion of the FDA. In addition, the START Pilot Program is in its first year as a pilot program, so there is no historical information on how that program is expected to be administered, and the stated intentions of the program may not be met, or the program may cease to have appropriate funding due to changes in the regulatory landscape. Moreover, neither participation in the START Pilot Program nor the receipt of either Fast Track designation or RMAT designation for a product candidate is any guarantee that there will be faster development or a faster or more streamlined regulatory review or approval process compared to products considered for approval under conventional FDA procedures. Neither participation in the START Pilot Program nor either Fast Track designation or RMAT designation will assure ultimate approval by the FDA. In addition, the FDA may later decide that the product candidates no longer meet the conditions to qualify for those programs, and we may not

receive the benefits of those programs for the relevant product candidate, or decide that the time period for FDA review or approval will not be shortened.

***We may seek certain designations for our product candidates, including Breakthrough Therapy and Priority Review designations by the FDA, however, even if we receive such designations, there is no guarantee that they would lead to faster development or regulatory review timelines or increase the likelihood of marketing approval for such product candidate.***

We may seek to have one or more of our products designated as a Breakthrough Therapy, which is defined as a product that is intended, alone or in combination with one or more other products, to treat a serious or life threatening disease or condition where preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For products that have been designated as Breakthrough Therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens.

We may seek an RMAT designation or a priority review designation for one or more of our product candidates. If the FDA determines that a product candidate qualifies as an RMAT, that is intended to treat, reverse, or cure a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the product has the potential to address unmet medical need for such a disease or condition, the FDA may grant participation in the RMAT program for that product candidate. If the FDA determines that a product candidate offers a treatment for a serious condition, and if approved, would provide a significant improvement in safety or effectiveness where no adequate therapy exists, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months.

As with the Fast Track designations and selection for participation in the START Pilot Program, these designations are within the discretion of the FDA. Even if we believe that one or more of our product candidates meets the criteria for these designations, the FDA may not agree and instead determine to not make such a designation. Even if one or more of our product candidates qualifies for either or both of these designations, the FDA may later decide that such product candidate no longer meets the conditions for those designations, and we may not receive the benefits of the designation for that product candidate. If a product candidate is awarded one or both designations by the FDA, it may not result in a faster or more streamlined regulatory review or approval process compared to products considered for approval under conventional FDA procedures, and it does not assure ultimate marketing approval of such product candidate by the FDA.

***We have received orphan drug designation for NGN-401 for the treatment of Rett syndrome, and we may seek orphan drug designation for certain future product candidates, but we may be unable to obtain such designations or to maintain the benefits associated with orphan drug designation, including market exclusivity, which may cause our revenue, if any, to be reduced.***

We have received orphan drug designation from the FDA and orphan drug designation and advanced therapy medicinal product designation from the European Medicines Agency (EMA) for NGN-401 for the treatment of Rett syndrome. Although we may seek orphan product designation for some or all of our other product candidates, we may never receive such designations. Under the Orphan Drug Act, the FDA may designate a drug or biological product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. Orphan drug designation must be requested before submitting a BLA. In the European Union, the EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the European Union would be

sufficient to justify the necessary investment in developing the drug or biological product or where there is no satisfactory method of diagnosis, prevention, or treatment, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and application fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA.

In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity for the orphan patient population. Exclusive marketing rights in the United States may also be unavailable if we or our collaborators seek approval for an indication broader than the orphan designated indication and may be lost if the FDA later determines that the request for designation was materially defective. In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following drug or biological product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Even with an orphan drug designation for our current and potential future product candidates, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain orphan drug exclusivity for an existing or future product candidate, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties still can be approved for the same condition even with an orphan drug designation. Even after an orphan drug is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug or biologic nor gives the drug or biologic any advantage in the regulatory review or approval process.

***We have received Rare Pediatric Disease designation by the FDA for NGN-401 for the treatment of Rett syndrome. However, Rare Pediatric Disease designation for any of our product candidates does not guarantee that the BLA for the product will qualify for a priority review voucher upon approval, and it does not lead to a faster development or regulatory review process, or increase the likelihood that our product candidates will receive marketing approval.***

Under the Rare Pediatric Disease Priority Review Voucher program, upon the approval of a qualifying BLA for the treatment of a rare pediatric disease, the sponsor of such an application would be eligible for a rare pediatric disease priority review voucher that can be used to obtain priority review for a subsequent BLA or NDA. If a product candidate is designated before September 30, 2024, it is eligible to receive a voucher if it is approved before September 30, 2026. While we have obtained Rare Pediatric Disease designation for NGN-401 for the treatment of Rett syndrome, it is unlikely that this product candidate will be approved by September 30, 2026. If approval is not obtained by then, we would not be in a position to obtain a priority review voucher, unless Congress further reauthorizes the program beyond the current sunset dates, which require that a product designated as being for a rare pediatric disease be approved by September 30, 2026. Additionally, designation of a biological product for a rare pediatric disease does not guarantee that a BLA will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Finally, a Rare Pediatric Disease designation does not lead to faster development or regulatory review of the product or increase the likelihood that it will receive marketing approval.



## General Risk Factors

***Our estimates of market opportunity and forecasts of market growth may prove to be inaccurate, and even if the markets in which we compete achieve the forecasted growth, our business may not grow at similar rates, or at all.***

Our market opportunity estimates and growth forecasts are subject to significant uncertainty and are based on assumptions and estimates which may not prove to be accurate. Our estimates and forecasts relating to size and expected growth of our target market may prove to be inaccurate. Even if the markets in which we compete meet our size estimates and growth forecasts, our business may not grow at similar rates, or at all. Our growth is subject to many factors, including our success in implementing our business strategy, which is subject to many risks and uncertainties.

Our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to obtain coverage and reimbursement and whether we own the commercial rights for that territory. If the number of our addressable patients is not as significant as we estimate, the indication approved by regulatory authorities is narrower than we expect or the treatment population is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved.

***We may become exposed to costly and damaging liability claims, either when testing a product candidate in the clinical or at the commercial stage, and our product liability insurance may not cover all damages from such claims.***

We are exposed to potential product liability and professional indemnity risks that are inherent in the research, development, manufacturing, marketing, and use of pharmaceutical products. While we currently have no products that have been approved for commercial sale, the current and future use of a product candidate in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims may be made by patients that use the product, healthcare providers, pharmaceutical companies, or others selling such product. Any claims against us, regardless of their merit, could be difficult and costly to defend and could materially and adversely affect the market for our products or any prospects for commercialization of our products. Although we believe we currently maintain adequate product liability insurance for NGN-401, NGN-101 and other product candidates, it is possible that our liabilities could exceed our insurance coverage or that in the future we may not be able to maintain insurance coverage at a reasonable cost or obtain insurance coverage that will be adequate to satisfy any liability that may arise. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

***Our manufacturing facility is located in Houston, Texas, making us vulnerable to risks (including weather-related risks) associated with maintaining those operations in a single geographic area.***

Our manufacturing facility is located in Houston, Texas, which is subject to extreme weather events such as hurricanes and other significant storms, which can cause interruption to our utilities and potentially result in damage to our facility, limit the ability of suppliers to reach us during such disruptions and adversely impact our manufacturing processes. For example, in July 2024, our facility in Houston sustained five days of power loss from the impact of Hurricane Beryl, which was a Category 1 hurricane. While we were able to maintain power to critical systems through the use of our generators, the outage caused a minor delay in our development activities and caused disruptions in our manufacturing processes, including in our clean rooms. The impact of Hurricane Beryl was not material to our operations, however, future weather events could cause more disruption, including the potential for a sustained loss of power that could result in costly delays to our manufacturing process or the loss of certain materials stored in our facility, which could in turn have a material adverse effect on our product development timeline and results of operations.

***Litigation costs and the outcome of litigation could have a material adverse effect on our business.***

From time to time we may be subject to litigation claims through the ordinary course of our business operations regarding, but not limited to, employment matters, security of patient and employee personal information,

contractual relations with collaborators and intellectual property rights. Litigation to defend ourselves against claims by third parties, or to enforce any rights that we may have against third parties, may continue to be necessary, which could result in substantial costs and diversion of our resources, causing a material adverse effect on our business, financial condition, results of operations or cash flows.

***Our business could be adversely affected by economic downturns, inflation, increases in interest rates, natural disasters, public health crises such as the COVID-19 pandemic, political crises, geopolitical events, such as conflicts between Russia and Ukraine and between Israel and the surrounding regions, or other macroeconomic conditions, which could have a material and adverse effect on our results of operations and financial condition.***

The global economy, including credit and financial markets, has experienced extreme volatility and disruptions, including, among other things, diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, supply chain shortages, increases in inflation rates, higher interest rates, and uncertainty about economic stability. For example, the COVID-19 pandemic resulted in widespread unemployment, economic slowdown and extreme volatility in the capital markets. The Federal Reserve has raised interest rates multiple times in response to concerns about inflation and it may not reduce interest rates in the near term or may raise them again. Higher interest rates, coupled with reduced government spending and volatility in financial markets, may increase economic uncertainty and affect consumer spending. Similarly, the ongoing military conflict between Russia and Ukraine, as well as the conflict between Israel and the surrounding regions, and rising tensions with China have created extreme volatility in the global capital markets and may have further global economic consequences, including disruptions of the global supply chain. Any such volatility and disruptions may adversely affect our business or the third parties on whom we rely. If the equity and credit markets deteriorate, including as a result of economic or political uncertainty, political unrest or war, it may make any necessary debt or equity financing more costly, more dilutive, or more difficult to obtain in a timely manner or on favorable terms, if at all. Increased inflation rates can adversely affect us by increasing our costs, including labor and employee benefit costs.

We may in the future experience disruptions as a result of such macroeconomic conditions, including delays or difficulties in initiating or expanding clinical trials and manufacturing sufficient quantities of materials. Any one or a combination of these events could have a material and adverse effect on our results of operations and financial condition.

### **Risks Related to Owning Our Stock**

***The market price of our common stock may continue to be volatile.***

The market price of our common stock following the merger has been and may continue to be subject to significant fluctuations. Some of the factors that may cause the market price of our common stock to fluctuate include:

- timing and results of clinical trials and preclinical studies of our product candidates, or those of our competitors or our existing or future collaborators;
- failure to meet or exceed financial and development projections that we may provide to the public;
- failure to meet or exceed the financial and development projections of the investment community;
- failure to achieve the perceived benefits of the merger as rapidly or to the extent anticipated by financial or industry analysts;
- announcements of significant acquisitions, strategic collaborations, joint ventures or capital commitments by us or our competitors;
- actions taken by regulatory agencies with respect to our product candidates, clinical studies, manufacturing process or sales and marketing terms;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;

- additions or departures of key personnel;
- significant lawsuits, including patent or stockholder litigation;
- if securities or industry analysts do not publish research or reports about our business, or if they issue adverse or misleading opinions regarding our business and stock;
- changes in the market valuations of similar companies;
- general market, macroeconomic or geopolitical conditions or market conditions in the pharmaceutical and biotechnology sectors;
- sales of securities by us or our securityholders in the future;
- if we fail to raise an adequate amount of capital to fund our operations or continued development of our product candidates;
- trading volume of our common stock;
- announcements by competitors of new commercial products, clinical progress or lack thereof, significant contracts, commercial relationships or capital commitments;
- adverse publicity relating to precision medicine product candidates, including with respect to other products in such markets;
- the introduction of technological innovations or new therapies that compete with our products; and
- period-to-period fluctuations in our financial results.

Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock. In addition, a recession, depression or other sustained adverse market event could materially and adversely affect our business and the value of our common stock. In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against such companies. Furthermore, market volatility may lead to increased shareholder activism if we experience a market valuation that activists believe is not reflective of our intrinsic value. Activist campaigns that contest or conflict with our strategic direction or seek changes in the composition of our board of directors could have an adverse effect on our operating results, financial condition and cash flows.

***We may be required to allocate resources to fulfilling the requirements of the CVR Agreement entered into in connection with the Reverse Merger (as defined below) related to certain legacy lease obligations, which may take away from our core programs and create a distraction for our management and employees.***

On December 18, 2023, we completed our business combination with our wholly owned subsidiary incorporated in the state of Nevada and also named Neurogene Inc. ("Neurogene OpCo") in accordance with the terms of the Agreement and Plan of Merger, dated as of July 17, 2023 (the "Merger Agreement"), by and among the Company, Project North Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of the Company ("Merger Sub"), and Neurogene OpCo, pursuant to which, among other matters, Merger Sub merged with and into Neurogene OpCo, with Neurogene OpCo surviving as a wholly owned subsidiary of the Company (the "Reverse Merger").

In connection with the Reverse Merger, we declared a dividend, to each person who, as of immediately prior to the effective time of the Reverse Merger, was a stockholder of the Company or had the right to receive our common stock pursuant to an existing pre-funded warrant, of the right to receive one non-transferable contingent value right (each, a "CVR") for each then outstanding share of our common stock (before giving effect to a 1-for-4 reverse stock split (the "Reverse Stock Split") that was implemented immediately prior to the effective time), each representing the non-transferable contractual right to receive certain contingent payments from the Company upon

the occurrence of certain events within agreed time periods. Holders of options to purchase our common stock outstanding immediately prior to the effective time of the merger will also receive four CVRs for each share of our common stock that may be issued upon exercise of such option, such that they will receive the same number of CVRs as they would have received if the option had been exercised before the Reverse Stock Split, subject to certain conditions set forth in the CVR Agreement.

Pursuant to the terms of the CVR Agreement, the holders of our common stock prior to the effective time of the Reverse Merger, including holders of existing pre-funded warrants and holders of options to purchase our common stock outstanding immediately prior to the effective time of the merger and exercised after the effective time of the merger, rather than all of our current holders of our common stock, are the primary recipients of any net proceeds of the disposition of the legacy assets related the business of Neoleukin Therapeutics, Inc. as it existed prior to the effective time of the Reverse Merger, the mitigation of legacy lease obligations related the business of Neoleukin Therapeutics, Inc. as it existed prior to the effective time of the Reverse Merger or receipt of any sales tax refund from the State of Washington based on tax returns filed by the Company prior to the effective time of the Reverse Merger. While we have entered into agreements for the disposition of certain legacy assets of Neoleukin, we are still pursuing a resolution of the legacy lease obligations of Neoleukin and expect that we will need to allocate resources, including payment of certain up-front costs, and time from employees and management to complete the resolution of such obligations and to administer the provisions of the CVR Agreement and distribution of any payments to holders of the CVRs.

Accordingly, we may be required to allocate a portion of our funds, time and resources to such activities and not our core programs and the foregoing could be a distraction to our management and employees. As a result, our operations and financial condition may be adversely affected.

***We have incurred, and will continue to incur additional costs and increased demands upon management as a result of complying with the laws and regulations affecting public companies.***

We have incurred and will continue to incur significant legal, accounting and other expenses as a public company that may not be reflected in our historical financial statements, which reflect our operation as a private company. Some of these additional expenses include costs associated with public company reporting obligations under the Securities Exchange Act of 1934, as amended (the “Exchange Act”). Our management team consists of our executive officers prior to the merger. These executive officers and other personnel will need to devote substantial time to complying with public company reporting requirements and compliance with applicable laws and regulations to ensure that we comply with all of these requirements. Any changes we make to comply with these obligations may not be sufficient to allow us to satisfy our obligations as a public company on a timely basis, or at all. These reporting requirements, rules and regulations, coupled with the increase in potential litigation exposure associated with being a public company, could also make it more difficult for us to attract and retain qualified persons to serve on the board of directors or on board committees or to serve as executive officers, or to obtain certain types of insurance, including directors’ and officers’ insurance, on acceptable terms.

***Once we are no longer a smaller reporting company or otherwise no longer qualify for applicable exemptions, we will be subject to additional laws and regulations affecting public companies that will increase our costs and the demands on management and could harm our operating results and cash flows.***

We are subject to the reporting requirements of the Exchange Act, which requires, among other things, that we file with the SEC annual, quarterly and current reports with respect to our business and financial condition as well as other disclosure and corporate governance requirements. We expect to still qualify as a “smaller reporting company,” as such term is defined in Rule 12b-2 under the Exchange Act, in at least the near term, which allows us to take advantage of many exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. Once we are no longer a smaller reporting company or otherwise no longer qualify for this exemption, we will be required to comply with these additional legal and regulatory requirements applicable to public companies and will incur significant additional legal, accounting and other expenses to do so. If we are not able to comply with the requirements in a timely manner or at all, our financial condition or the market price of our common stock may be harmed. For example, if we or our

independent auditor identifies deficiencies in our internal control over financial reporting that are deemed to be material weaknesses, we could face additional costs to remedy those deficiencies, the market price of our stock could decline or we could be subject to sanctions or investigations by the SEC or other regulatory authorities, any of which would require additional financial and management resources.

***If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.***

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act and the rules and regulations of Nasdaq. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. We must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in each Annual Report on Form 10-K, as required by Section 404 of the Sarbanes-Oxley Act. Prior to the merger in December 2023, our operating and finance teams were part of a private company, and therefore were not previously required to test internal controls within a specified period. As a result, we have incurred and may continue to incur substantial professional fees and internal costs to expand our accounting and finance functions as well as to expend significant management efforts. We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

For example, our internal controls related to the cash disbursements process were not adequately designed to identify unauthorized payment requests, resulting in the identification of a material weakness. Specifically, at the end of August 2023, we discovered that we were subject to a business email compromise attack by a third party. This deficiency in our controls resulted in the diversion of payments to a fraudulent bank account. While management has determined in its assessment of our internal control over financial reporting as of December 31, 2023, that we have remediated this material weakness, there can be no assurance that the remediation will prevent similar attacks in the future or that we will not identify other material weaknesses in the future. If we are unable to successfully remediate a material weakness in our internal control over financial reporting, or if we identify any other material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements, investors may lose confidence in our financial reporting, and our stock price may decline as a result. We also could become subject to investigations by Nasdaq, the SEC or other regulatory authorities.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our common stock could decline and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities.

***Our certificate of incorporation and bylaws, as well as provisions under Delaware law, could make an acquisition of the company more difficult and may prevent attempts by our stockholders to replace or remove management.***

Provisions in our certificate of incorporation and bylaws may discourage, delay or prevent a merger, acquisition or other change in control of the company that stockholders may consider favorable, including transactions in which our common stockholders might otherwise receive a premium price for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors will be responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our

stockholders to replace or remove current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that all members of the board are not elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call a special meeting of stockholders;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 66 2/3% of the votes that all stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the DGCL, which prohibits stockholders owning in excess of 15% of our outstanding voting stock from merging or combining with us. Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirors to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders.

***Our governing documents provide that, unless we consent in writing to the selection of an alternative forum, certain designated courts will be the sole and exclusive forum for certain legal actions between us and our stockholders, which could limit our stockholders’ ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or agents.***

Our governing documents provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware is the sole and exclusive forum for state law claims for (i) any derivative action or proceeding brought on the company’s behalf, (ii) any action asserting a claim of or based on a breach of a fiduciary duty owed by any of our current or former directors, officers, or other employees to the company or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the DGCL, the certificate of incorporation or the bylaws, (iv) any action to interpret, apply, enforce or determine the validity of the certificate of incorporation or bylaws, or (v) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein, which for purposes of this risk factor refers to herein as the “Delaware Forum Provision.” Our governing documents further provide that, unless we consent in writing to an alternative forum, the federal district courts of the United States will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act of 1933, as amended (the “Securities Act”), which for purposes of this risk factor refers to herein as the “Federal Forum Provision.” Neither the Delaware Forum Provision nor the Federal Forum Provision will apply to any causes of action arising under the Exchange Act. In addition, any person or entity purchasing or otherwise acquiring any interest in shares of our capital stock will be deemed to have notice of and consented to the foregoing Delaware Forum Provision and Federal Forum Provision; *provided*, however, that stockholders cannot and will not be deemed to have waived our compliance with the U.S. federal securities laws and the rules and regulations thereunder.

The Delaware Forum Provision and the Federal Forum Provision may impose additional litigation costs on our stockholders in pursuing any such claims, particularly if such stockholders do not reside in or near the State of Delaware. Additionally, these forum selection clauses may limit our stockholders’ ability to bring a claim in a

judicial forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders.

***Future sales of shares by existing stockholders could cause our stock price to decline.***

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the trading price of our common stock could decline. Based on shares outstanding as of November 13, 2024, there are approximately 20,979,721 shares of our common stock outstanding or issuable on exercise of pre-funded warrants to purchase common stock. All outstanding shares of common stock and any shares issuable on exercise of pre-funded warrants or vested options to purchase our common stock, other than (a) shares held by our affiliates or otherwise subject to restrictions on vesting and exercise, and (b) certain shares held by the Company's directors that are subject to lock-up agreements entered into in connection with the November private placement that expire in December 2024 are freely tradable, without restriction, in the public market. If a significant number of these shares are sold, the trading price of our common stock could decline.

***Our executive officers, directors and principal stockholders have the ability to control or significantly influence all matters submitted to our stockholders for approval.***

Our executive officers, directors and principal stockholders beneficially own a significant percentage of our outstanding common stock. As a result, if these stockholders were to choose to act together, they would be able to control or significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these stockholders, if they choose to act together, would control or significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent our acquisition on terms that other stockholders may desire.

***We may be exposed to increased litigation, including stockholder litigation, which could have an adverse effect on our business and operations.***

We may be exposed to increased litigation from stockholders, suppliers and other third parties, which may have an adverse impact on our business and results of operations or may cause disruptions to our operations. In the past, stockholders have initiated class action lawsuits against biotechnology companies following periods of volatility in the market prices of these companies' stock, and we may also be subject to threats of litigation based on our recent merger activity. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources, which could have a material adverse effect on our business, financial condition and results of operations.

***If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.***

The trading market for our common stock will be influenced by the research and reports that equity research analysts publish about us and our business. Equity research analysts may elect to not provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. If we do have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our common stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of us or fails to publish reports on us regularly, demand for our common stock could decrease, which in turn could cause our stock price or trading volume to decline.

***We may be subject to adverse legislative or regulatory tax changes that could negatively impact our financial condition.***

The rules dealing with U.S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or our stockholders. We

continue to assess the impact of various tax reform proposals and modifications to existing tax treaties in all jurisdictions where we have operations or employees to determine the potential effect on our business and any assumptions we make about our future taxable income. We cannot predict whether any specific proposals will be enacted, the terms of any such proposals or what effect, if any, such proposals would have on our business if they were to be enacted. For example, the United States recently enacted the Inflation Reduction Act of 2022, which implements, among other changes, a 1% excise tax on certain stock buybacks. In addition, beginning in 2022, the Tax Cuts and Jobs Act eliminated the option to deduct research and development expenditures and requires taxpayers to amortize them generally over five years. The U.S. Congress is considering legislation that would restore the current deductibility of research and development expenditures; however, there is no assurance that the current provision will be repealed or otherwise modified. Such changes, among others, may adversely affect our effective tax rate, results of operation and general business condition.



## USE OF PROCEEDS

We are not selling any securities under this prospectus and we will not receive any proceeds from the sale of the Resale Shares covered hereby. The net proceeds from the sale of the Resale Shares offered by this prospectus will be received by the Selling Stockholders. Some of the shares of Common Stock offered hereby are issuable upon the exercise of the Pre-Funded Warrants. Upon exercise of such Pre-Funded Warrants for cash, we will receive the nominal cash exercise price paid by the holders of the Pre-Funded Warrants. We intend to use those proceeds, if any, for general corporate purposes.

Subject to limited exceptions, the Selling Stockholders will pay any underwriting discounts and commissions and expenses incurred by the Selling Stockholders for brokerage, accounting, tax or legal services or any other expenses incurred by the Selling Stockholders in disposing of any of the Resale Shares. We will bear the costs, fees and expenses incurred in effecting the registration of the Resale Shares covered by this prospectus, including all registration and filing fees, Nasdaq listing fees and fees and expenses of our counsel and our independent registered public accounting firm.

## UNAUDITED PRO FORMA FINANCIAL INFORMATION

### Introduction

The following unaudited pro forma financial information has been prepared to further your understanding of the financial impacts of the December 18, 2023 merger (the “Reverse Merger”) between (1) Neurogene Inc., a Delaware corporation (formerly known as Neoleukin Therapeutics, Inc., or “Neoleukin”), (2) Project North Merger Sub, Inc., a Delaware corporation and wholly owned subsidiary of Neoleukin (“Merger Sub”), and (3) Neurogene Inc., a Nevada corporation (“Neurogene OpCo”, formerly a Delaware corporation). On July 18, 2023, Neoleukin entered into an Agreement and Plan of Merger (the “Merger Agreement”) with Merger Sub and Neurogene OpCo. Hereinafter, Neoleukin and Neurogene OpCo are collectively referred to as the “companies,” and the companies, subsequent to the Reverse Merger, are referred to herein as the “combined company.”

On December 18, 2023 (the “Closing Date”), Neoleukin and Neurogene OpCo completed the Reverse Merger through the exchange of Neoleukin common stock and pre-funded warrants for all of Neurogene OpCo’s issued and outstanding shares of Neurogene OpCo common stock and Neurogene OpCo pre-funded warrants, respectively, including the shares and pre-funded warrants issued in connection with the conversion of Neurogene OpCo preferred stock and the Neurogene OpCo pre-closing financing, respectively (the “Closing”). Additionally, a 1-for-4 reverse stock split of Neoleukin common stock was effected on the Closing Date (the “Reverse Stock Split”). Refer to the subsequent section titled “Description of the Transactions” for additional details.

The pro forma financial information that follows has been prepared to show the financial statement impacts of the Reverse Merger and Neurogene OpCo pre-closing financing (collectively, the “Transactions”) assuming that the Transactions had occurred on January 1, 2023. As the Transactions were consummated or completed on the Closing Date, these transactions have been recorded in Neurogene Inc.’s financial statements as of and for the year ended December 31, 2023. In addition, since the Reverse Merger has been accounted for as a reverse asset purchase (as described in the subsequent section titled “Description of the Transactions”), Neurogene Inc.’s financial statements as of and for the year ended December 31, 2023 reflect the historical financial statements of Neurogene OpCo for such periods. Since Neurogene Inc.’s consolidated balance sheet as of December 31, 2023, as included elsewhere in this prospectus, reflects the full effect of the Transactions, no pro forma adjustments are required with respect to the combined company’s consolidated balance sheet as of such date. Additionally, since Neurogene Inc.’s consolidated statement of operations for the nine months ended September 30, 2024, as included elsewhere in this prospectus, reflects the full effect of the Transactions for the entire nine-month period, no pro forma adjustments are required with respect to the combined company’s consolidated statement of operations for the same period. Accordingly, the pro forma financial information that follows only gives effect to the incremental impact that certain aspects of the Transactions would have had on the combined company’s net loss and net loss per share for the year ended December 31, 2023, if the Transactions had been consummated on January 1, 2023, rather than the Closing Date.

## 1. Description of the Transactions

### *The Reverse Merger*

On the Closing Date, Neoleukin consummated the previously announced reverse asset purchase pursuant to the terms of the Merger Agreement, dated July 18, 2023, by and among Neoleukin, Merger Sub, and Neurogene OpCo. Pursuant to the terms of the Merger Agreement, Merger Sub merged with and into Neurogene OpCo, with Neurogene OpCo continuing as a wholly-owned subsidiary of Neoleukin. After the completion of the Reverse Merger, Neoleukin changed its corporate name to “Neurogene Inc.” Upon Closing, the business of Neurogene OpCo continued as the business of the combined company.

Additionally, subject to the terms and conditions of the Merger Agreement, at the Closing (or immediately prior to, where indicated), the following activities occurred and were reflected as of the date of occurrence in Neurogene Inc’s financial statements as of and for the year ended December 31, 2023 and, accordingly, did not require the recognition of pro forma adjustments:

- Immediately prior to Closing, Neoleukin effected the Reverse Stock Split.
- All of the then outstanding shares of Neurogene OpCo Class A common stock were converted into 3,240,888 shares of Neoleukin common stock, based on an exchange ratio of approximately 0.0756 (the “Exchange Ratio”).
- All of the then outstanding shares of Neurogene OpCo preferred stock were converted into 7,231,747 shares of Neoleukin common stock, based on the Exchange Ratio.
- Each then outstanding Neurogene OpCo stock option granted under Neurogene OpCo’s 2018 Equity Incentive Plan was exchanged for an equivalent stock option of Neoleukin, adjusted to reflect the Exchange Ratio as necessary. Such assumed options continue to be governed by the terms and conditions of Neurogene OpCo’s 2018 Equity Incentive Plan.
- Each then outstanding Neurogene OpCo pre-funded warrant to purchase shares of Neurogene OpCo common stock was converted into a pre-funded warrant to purchase shares of Neoleukin common stock, adjusted to reflect the Exchange Ratio.
- The payment of transaction costs was incurred by both Neoleukin and Neurogene OpCo.

Immediately after the Reverse Merger, the combined company had pre-funded warrants outstanding to purchase an aggregate of 4,063,361 shares of Neoleukin common stock. Of the total pre-funded warrants outstanding, 425,987 pre-funded warrants are owned by Neoleukin securityholders, 1,825,635 pre-funded warrants are owned by legacy Neurogene OpCo securityholders and 1,811,739 pre-funded warrants are owned by investors who participated in the Neurogene OpCo pre-closing financing. The pre-funded warrants owned by legacy Neoleukin securityholders are exercisable at any time for an exercise price of \$0.00002 and the remainder of the pre-funded warrants are exercisable at any time for an exercise price of \$0.000001, in each case subject to certain beneficial ownership limitations.

Immediately after the Reverse Merger, Neoleukin securityholders owned approximately 16% of the outstanding shares of capital stock of the combined company, legacy Neurogene OpCo securityholders, excluding shares of Neurogene OpCo common stock and Neurogene OpCo pre-funded warrants purchased in the Neurogene OpCo pre-closing financing, own approximately 57% of the outstanding shares of the combined company, and holders of shares of Neurogene OpCo common stock and Neurogene OpCo pre-funded warrants issued in the Neurogene OpCo pre-closing financing own approximately 27% of the outstanding shares of capital stock of the combined company.

Based on the following factors, the Reverse Merger was accounted for as a reverse asset acquisition under the Accounting Standards Codification (“ASC”) 805, *Business Combinations* (“ASC 805”):

- Neurogene OpCo stockholders owned approximately 84% of the voting rights in the combined company, and thus, had sufficient voting rights to exert influence over the Company;

- Neurogene OpCo designated a majority of the combined company’s board of directors and maintained a majority of the composition of management; and
- At the time of Closing, Neoleukin did not meet the definition of a business but it did represent a group of assets.

The Reverse Merger was accounted for as a reverse asset purchase, with any difference between the consideration transferred in the merger and the fair value of the net assets acquired allocated to any non-monetary assets on a pro-rata basis. As the fair value of Neoleukin’s net assets acquired at Closing exceeded the total consideration transferred after the fair value allocation, (i) \$1.3 million of the difference was recorded as a contingent consideration liability and reflected on the combined company’s December 31, 2023 consolidated balance sheet for payments that are probable and estimable under a Contingent Value Rights Agreement described below and (ii) the remaining \$16.4 million was recognized as a bargain purchase gain and reflected on the combined company’s consolidated statement of operations for the year ended December 31, 2023. The bargain purchase gain was not adjusted to give pro forma effect to the assumption that the Reverse Merger occurred as of January 1, 2023, rather than December 18, 2023 because management believes that reflecting the actual result of the bargain purchase gain in the following pro forma information provides more meaningful information and a complete understanding of the impact of the Reverse Merger on the combined company’s consolidated statements of operations for the year ended December 31, 2023.

Under the terms of the Merger Agreement, prior to the Closing, the board of directors of Neoleukin accelerated the vesting of (i) each unexercised and unvested Neoleukin option that has an exercise price per share less than \$75.60, after giving effect to the Reverse Stock Split that was effected on December 18, 2023, that was held by a current employee, director or consultant of Neoleukin as of immediately prior to the effective time (or who ceased to be a current employee, director or consultant of Neoleukin as of immediately prior to the effective time) and (ii) restricted stock units that vested solely on the basis of time, in each case, in accordance with the terms of the Merger Agreement. In addition, two Neoleukin executives were entitled to extended option exercise periods upon a change in control, as defined, pursuant to the terms of their employment agreements, as amended. The terms of the employment agreements were negotiated prior to entering into negotiations for the Reverse Merger with Neurogene OpCo. The (i) acceleration of vesting of the Neoleukin options and restricted stock units and (ii) extension of the option exercise periods were treated as a modification of the awards. The incremental fair value associated with the modification was immaterial and therefore was not included as a pro forma adjustment in the combined company’s consolidated statements of operations for the year ended December 31, 2023.

The pre-merger employment agreements for the two Neoleukin executives also included severance, bonus and retention payments, the aggregate of which were treated as pre-combination compensation expense of Neoleukin and were included in the liabilities assumed by Neurogene OpCo upon Closing. In addition, certain non-executive Neoleukin employees entered into separation agreements prior to merger negotiations with Neurogene OpCo, pursuant to which they were entitled to severance, bonus, and retention payments. These payments were treated as pre-combination compensation expense of Neoleukin and were included in the liabilities assumed by Neurogene OpCo upon Closing. These transactions have been recorded at Closing and reflected on the combined company’s consolidated financial statements as of and for the year ended December 31, 2023.

#### ***Contingent Value Rights Agreement***

On December 18, 2023, immediately prior to Closing, Neoleukin entered into a contingent value rights agreement (the “CVR Agreement”) with a rights agent and the lease representative, pursuant to which holders of Neoleukin common stock or pre-funded warrants prior to Closing received one nontransferable contingent value right (each, a “CVR”) for each outstanding share of Neoleukin common stock held by such stockholder or warrant holder immediately prior to Closing. Holders of options to purchase Neoleukin common stock outstanding immediately prior to the effective time of the Reverse Merger who elect to exercise those options following the Reverse Merger will also receive one CVR for each share of Neoleukin common stock issued upon exercise of such option, subject to certain conditions set forth in the CVR Agreement. Each CVR represents the contractual right to receive (i) certain net savings, if any, realized by the Company by June 30, 2029 in connection with Neoleukin’s legacy lease obligations (the “Lease CVR”), including those related to Neoleukin’s sublease, entered into in October

2023, (ii) 100% of net proceeds, if any, derived from any consideration paid as a result of the sale of Neoleukin’s pre-merger legacy assets pursuant any agreements entered into before Closing, and 80% of net proceeds, if any, derived from any consideration paid as a result of the sale of Neoleukin’s pre-merger legacy assets pursuant any agreements entered into within one year after Closing (the “Intellectual Property CVR”), and (iii) certain net proceeds, if any, derived from Neoleukin’s anticipated sales tax refund from Washington State relating to tax returns filed by Neoleukin prior to Closing (the “Sales Tax CVR”). At Closing, a \$1.3 million contingent consideration liability was recorded and reflected on the combined company’s consolidated balance sheet as of December 31, 2023, for payments that were probable and estimable under the Lease CVR. As no other amounts related to the CVR Agreement were probable as of Closing, no contingencies for the Intellectual Property CVR or Sales Tax CVR have been recorded.

### ***Neurogene OpCo Pre-Closing Financing***

Concurrently with the execution and delivery of the Merger Agreement, and in order to provide Neurogene OpCo with additional capital for its development programs, Neurogene OpCo entered into a subscription agreement (the “Subscription Agreement”) with certain investors. Pursuant to the terms of the Subscription Agreement, immediately prior to Closing, Neurogene OpCo issued and sold to the investors: (i) 2,792,206 shares of Neurogene OpCo common stock and (ii) 1,811,739 pre-funded warrants, exercisable for 1,811,739 shares of Neurogene OpCo common stock, at a purchase price of approximately \$20.63 per share or \$20.63 per warrant after reflecting the Exchange Ratio, for an aggregate purchase price of approximately \$95.0 million, before \$6.9 million in transaction costs incurred related to the Neurogene OpCo pre-closing financing. At Closing, shares of Neurogene OpCo common stock and Neurogene OpCo pre-funded warrants issued pursuant to the Neurogene OpCo pre-closing financing were converted into the right to receive shares of common stock and pre-funded warrants, respectively, of Neoleukin, adjusted for the Exchange Ratio. The combined company classified the Neurogene OpCo pre-funded warrants within permanent equity. The Neurogene OpCo pre-closing financing was recorded and reflected on the combined company’s consolidated balance sheet as of December 31, 2023.

## **2. Basis of Pro Forma Presentation**

Due to the fact that (1) the effects of the Reverse Merger and Neurogene OpCo pre-closing financing were fully reflected in Neurogene Inc’s consolidated balance sheet and consolidated statement of operations as of and for the year ended December 31, 2023; (2) at the time of Closing, Neoleukin did not meet the definition of a business under ASC 805 but it did represent a group of assets; (3) the Reverse Merger was accounted for as a reverse asset acquisition, with any difference between the consideration to be transferred in the Reverse Merger and the fair value of the net assets acquired allocated to any non-monetary assets on a pro-rata basis; and (4) Neurogene Inc’s historical financial statements reflected the historical financial information, inclusive of the historical financial position and historical results of operations, of Neurogene OpCo for the equivalent reporting periods, no pro forma adjustments have been deemed necessary to give effect to the Transactions, as if they had occurred as of January 1, 2023 rather than December 18, 2023, in Neurogene Inc’s consolidated statements of operations for the year ended December 31, 2023. As subsequently presented and discussed in greater detail, the reported basic and diluted net loss per share for the year ended December 31, 2023 was adjusted to give pro forma effect to the assumption that the issuances of Neoleukin common stock and pre-funded warrants to consummate the Reverse Merger and Neurogene OpCo pre-closing financing occurred as of January 1, 2023, rather than December 18, 2023. No pro forma adjustments were included to reflect any management adjustments or any synergies, operating efficiencies, or other benefits that may have resulted from consummation of the Transactions.

In accordance with Rule 11-02(a)(1) of Regulation S-X, in circumstances where a limited number of pro forma adjustments are required and those adjustments are easily understood, a narrative description of the pro forma effects of a transaction may be provided in lieu of a pro forma condensed balance sheet, pro forma condensed statements of comprehensive income, and accompanying explanatory notes. Due to the fact that no pro forma adjustments were deemed necessary to give effect to the Transactions as if they had occurred as of January 1, 2023, management has elected to prepare the narrative disclosures that follow to illustrate the pro forma effects of the aforementioned transactions on Neurogene Inc’s net loss per share amounts reported for the year ended December 31, 2023. The narrative pro forma disclosures that follow were derived from, and should be read in connection with the audited

consolidated financial statements of Neurogene Inc. as of and for the year ended December 31, 2023, as included elsewhere in this prospectus.

The unaudited pro forma financial information that follows also should be read together with the section of this prospectus titled “*Management’s Discussion and Analysis of Financial Condition and Results of Operations.*”

The unaudited pro forma financial information has been prepared for illustrative purposes only and does not purport to represent, and is not necessarily indicative of, what the actual reported net loss per share of Neurogene Inc. would have been for the year ended December 31, 2023 had the Transactions taken place on January 1, 2023. This information also should not be deemed indicative of what the results of operations of Neurogene Inc. will be for any future period.

### **3. Narrative Discussion of Pro Forma Adjustments to the Combined Company’s Reported Net Loss and Net Loss Per Share**

There were no pro forma adjustments to Neurogene Inc.’s reported net loss of \$36.3 million for the year ended December 31, 2023.

The pro forma basic and diluted net loss per share has been adjusted to reflect the Transactions, including the exchange and issuances of Neoleukin common stock and pre-funded warrants in connection with the Reverse Merger and Neurogene OpCo pre-closing financing, respectively, after giving effect to the Reverse Stock Split that was effected on December 18, 2023, as if such transactions had occurred on January 1, 2023. Therefore, the reported annual net loss of \$36.3 million, rather than the net income of \$13.7 million for the post-merger period presented in combined company’s consolidated statements of operations for the year ended December 31, 2023, was used as the numerator for the calculation of the pro forma basic and diluted net loss per share. The components of the denominator for the calculation of the pro forma basic and diluted net loss per share include (1) weighted-average Neurogene OpCo common shares outstanding of 426,097, and (2) the aggregated 12,374,983 shares of Neoleukin common stock and 4,063,361 of the Neoleukin pre-funded warrants issued in connection with the Reverse Merger and Neurogene OpCo pre-closing financing, assuming the Transactions had occurred as of January 1, 2023, rather than December 18, 2023, resulting in the pro forma basic and diluted net loss per share of \$2.15 for the year ended December 31, 2023. Since these pre-funded warrants are issuable for little or no consideration, management considered them outstanding in the context of pro forma basic net loss per share.

Potentially dilutive shares have been deemed to be anti-dilutive and, accordingly, have been excluded from the calculation of diluted net loss per share. Potentially dilutive shares that have been excluded from the determination of diluted net loss per share include approximately 823,833 options to purchase shares of Neoleukin common stock upon consummation of the Reverse Merger for the year ended December 31, 2023.

## MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

*The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our audited consolidated financial statements and related notes appearing elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business, include forward-looking statements that involve risks, uncertainties, and assumptions. As a result of many factors, including those factors set forth in the section entitled "Risk Factors," our actual results could differ materially from the results described in or implied by these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in the section of this report entitled "Risk Factors." You should carefully read the "Cautionary Note About Forward-Looking Statements" and "Risk Factors" sections of this prospectus to gain an understanding of the important factors that could cause actual results to differ materially from the results described below.*

*In this section, references to "we," "our," "us," and "the Company" refer to pre- and post-merger Neurogene Inc., unless otherwise indicated.*

### Overview

Despite recent scientific advances in genetics, most neurological diseases, particularly those with devastating consequences to patients, are left untreated. Conventional gene therapy is an attractive potential treatment approach for only a limited number of monogenic diseases due to the challenges caused by the complex biology of neurological diseases and by inherent variable transgene uptake and expression. We are a clinical-stage biotechnology company committed to overcoming these limitations and turning today's complex devastating neurological diseases into treatable conditions. By harnessing our proprietary transgene regulation technology, EXACT™ (Expression Attenuation via Construct Tuning), we are building a robust and differentiated product portfolio of genetic medicines for rare neurological diseases with high unmet need not otherwise addressable by conventional gene therapy. Our EXACT approach leverages key scientific breakthroughs, including gene transfer technology, microRNA-based genetic circuits, and adeno-associated virus ("AAV") delivery, and is designed to deliver therapeutic levels of transgene to key areas of the brain that underlie neurological disease pathology.

Our first clinical-stage program to utilize the EXACT platform is NGN-401, which is in development for the treatment of Rett syndrome, a disease with a patient population that has a significant unmet need, and that ultimately progresses to substantial neurological and physical impairment and premature death. We are conducting a Phase 1/2 open-label, multi-center clinical trial of NGN-401 gene therapy for the treatment of female patients with classic Rett syndrome that is assessing the safety, tolerability, and efficacy of NGN-401 at a dose of 1E15 vg. In November 2024, we announced the initiation of an adolescent/adult cohort that will evaluate NGN-401 in participants ages 16 and above. Clinical grade NGN-401 was manufactured at our manufacturing facility and is being used for dosing in the Phase 1/2 clinical trial. NGN-401 is delivered using a one-time intracerebroventricular ("ICV") procedure, which we believe is the most suitable route of administration to achieve optimal biodistribution in key regions of the brain and other parts of the nervous system.

The NGN-401 clinical trial has been initiated in three regions - the United States, the United Kingdom ("UK") and Australia, after clearance of our Investigational New Drug ("IND") application by the U.S. Food and Drug Administration ("FDA") in January 2023, the clearance of the clinical trial application by the UK Medicines and Healthcare Products Regulatory Agency in January 2024, and acknowledgement from the Australian Therapeutic Goods Administration and approval from the Human Research Ethics Committee in May 2024. Participants have been dosed in all three regions.

On November 11, 2024, we announced positive interim clinical data in all participants in the 1E15 vg cohort (low-dose cohort, n=4) from the Phase 1/2 clinical trial with a data cut-off date of October 17, 2024. Consistent, concordant and durable improvements were observed as measured from baseline across multiple Rett syndrome clinical assessments. Participants also achieved meaningful gains of skills and developmental milestones in hand function, gross motor, and communication, all of which are core clinical domains of Rett syndrome. These gains of

skills and developmental milestones would not be expected to occur when compared and contextualized against the natural history of Rett syndrome. Additionally, objective improvements in autonomic function were recorded.

Consistent improvements were observed in the following:

	CGI-I		CGI-S Total Score		RSBQ		Gain of Skills, Developmental Milestones and Symptom Improvement in RTT Clinical Domains				
	Improved?	How many points?*	Improved?	How many points?	Improved?	How many points? (% Change)	Hand Function	Gross Motor	Communication	Autonomic	Attentiveness
LD:1 15 mos. post-NGN-401	✓	2 pts.			✓	10 pts. (-28%)	✓	✓	✓	✓	✓
LD:2 12 mos. post-NGN-401	✓	2 pts.	✓	1 pt.	✓	32 pts. (-52%)	✓	✓	✓	✓	✓
LD:3 9 mos. post-NGN-401	✓	2 pts.			✓	5 pts. (-29%)	✓	✓		✓	✓
LD:4 3 mos. post-NGN-401	✓	2 pts.			✓	8 pts. (-28%)	✓			✓	✓

As of data cut-off date of October 17, 2024

\*Each participant achieved a 2-point improvement or “much improved” from baseline.

CGI-I = Clinician Global Impression-Improvement; CGI-S = Clinician Global Impression-Severity; RSBQ = Rett Syndrome Behavior Questionnaire

We also announced safety and tolerability data from the Phase 1/2 clinical trial with a data cut-off date of October 17, 2024. NGN-401 has been well-tolerated and shows a favorable safety profile at a dose of 1E15 vg (n=5, low-dose cohort), with no treatment-related serious adverse events (“SAEs”) at that dose. Treatment-related AEs at the 1E15 vg dose have been mild/Grade 1. One Grade 1 AE of abnormal sural (sensory) nerve conduction study at the 1E15 vg dose level was observed, and the participant is asymptomatic. Treatment-related AEs in the first two participants dosed with 3E15 vg of NGN-401 (high-dose cohort) included Grade 2 and Grade 3 elevations in alanine aminotransferase and aspartate aminotransferase levels, and Grade 2 decreased platelets, which resolved with steroids. One Grade 1 AE of abnormal sural (sensory) nerve conduction study at the 3E15 vg dose level was observed, and the participant is asymptomatic. Across both dose levels, there were no signs or symptoms indicating MeCP2 overexpression toxicity, and there were no ICV procedure-related AEs.

On November 11, 2024, we were notified of an SAE in the third participant who was dosed with 3E15 vg (high-dose cohort) on November 5, 2024. The participant subsequently died following complications from a rare and life-threatening hyperinflammatory syndrome associated with systemic exposure to high doses of AAV. Upon notification of the SAE, the Company consulted with the Data Safety Monitoring Board (DSMB) regarding the continued dosing of participants in the 1E15 vg (low-dose cohort). Following this consultation, the DSMB did not express concerns regarding the continued dosing of participants in the low-dose cohort. The Company also paused further use of the 3E15 vg dose (high dose cohort) on November 11, 2024 and does not plan to enroll any further participants at that dose level. The U.S. Food and Drug Administration (“FDA”) completed a review of the safety data for NGN-401 and allowed us to proceed with the Phase 1/2 trial using the 1E15 vg dose for both the pediatric and adolescent/adult cohorts. We are updating the trial protocol to remove the 3E15 vg dose. We will also incorporate the 1E15 vg dose in our future registrational trial design planning.

The baseline demographics of the first five participants who received NGN-401 in the 1E15 vg (low-dose) Cohort 1, and the first two participants who received NGN-401 in the 3E15 vg (high-dose) in Cohort 2 include:

	Low-Dose Cohort 1 (1E15 vg)					High-Dose Cohort 2 (3E15 vg)	
	Participant 1 (LD:1)	Participant 2 (LD:2)	Participant 3 (LD:3)	Participant 4 (LD:4)	Participant 5 (LD:5)	Participant 1 (HD:1)	Participant 2 (HD:2)
<b>Age at Dosing in Years</b>	7	4	6	7	6	5	7
<b>MECP2 Mutation Severity</b>	Mild	Severe	Severe	Severe	Severe	Severe	Unclassified
<b>Baseline Disease Severity as Indicated by CGI-S Score</b>	4 (moderately ill)	5 (markedly ill)	5 (markedly ill)	5 (markedly ill)	5 (markedly ill)	5 (markedly ill)	4 (moderately ill)
<b>Time Post Treatment with NGN-401 in Months</b>	~15	~12	~9	<6	~1	~5	~2

In November 2024, we also shared that we have gained alignment with the FDA on our potency assay strategy and CMC scale-up planning for the program. We expect to share information about our registration trial design in the first half of 2025 and to announce additional Phase 1/2 clinical data in the second half of 2025.

In June 2024, we also announced that NGN-401 was one of four sponsors selected by the Center for Biologics Evaluation and Research at the FDA to participate in the FDA’s Support for clinical Trials Advancing Rare disease Therapeutics (“START”) Pilot Program based on potential for clinical benefits and clinical development program readiness. As part of the START Program, we have opportunities for enhanced communications with the FDA, with the aim to further accelerate the pace of NGN-401’s development. These opportunities are designed to provide frequent advice and regular ad-hoc conversations to address product-specific development issues, including, but not limited to, clinical study design, choice of control group and fine-tuning the choice of patient population. In August 2024, we announced that NGN-401 also received Regenerative Medicine Advanced Therapy (“RMAT”) designation from the FDA. RMAT designation is granted for regenerative medicines intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition, and with preliminary clinical evidence that indicates that the drug has the potential to address unmet medical needs. Benefits of the RMAT designation program include all the benefits of Fast Track and Breakthrough Therapy designation programs, including early and frequent communications with FDA senior managers, intensive guidance on efficient drug development and eligibility for an Accelerated Approval pathway and Priority Review.

We believe that our EXACT platform has broad applicability in complex neurological diseases not otherwise easily addressable by conventional gene therapy. In addition to our Rett syndrome program, we have multiple programs in the discovery stage. We anticipate advancing one program into clinical development in 2025.

In addition to NGN-401, we have also been pursuing a conventional gene therapy program in an ongoing Phase 1/2 clinical trial of NGN-101 for the treatment of CLN5 Batten disease. This patient population has a significant unmet need and experiences extensive neurological and physical impairment leading to blindness, loss of motor function and early mortality. Our Phase 1/2 clinical trial of NGN-101 was the first trial to assess the treatment of both neurodegenerative and ocular disease manifestations of Batten disease. In November 2024, we announced that the Company does not expect to move forward with the NGN-101 CLN5 Batten disease gene therapy program at this time. Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. We are currently evaluating options for the program.



We also established a fully operational current good manufacturing practices (“cGMP”) facility in Houston, Texas used to manufacture current and future product for research, toxicology and clinical studies. We believe that our in-house manufacturing capabilities better enable control of product quality and development timelines, strategic pipeline and financial flexibility, and clinical-to-commercial continuity.

### **Completion of the Reverse Merger and Pre-Closing Financing**

On December 18, 2023, we completed our business combination with Neurogene OpCo (the “Closing”) in accordance with the terms of the Agreement and Plan of Merger, dated as of July 17, 2023 (the “Merger Agreement”), by and among the Company, Project North Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of the Company (“Merger Sub”), and Neurogene OpCo, pursuant to which, among other matters, Merger Sub merged with and into Neurogene OpCo, with Neurogene OpCo surviving as a wholly owned subsidiary of the Company (the “Reverse Merger”). In connection with the completion of the Reverse Merger, the Company changed its name from “Neoleukin Therapeutics, Inc.” (“Neoleukin”) to “Neurogene Inc.,” and the business conducted by the Company became primarily the business conducted by Neurogene OpCo. Immediately prior to Closing, the Company effected a 1-for-4 reverse stock split (the “Reverse Stock Split”). Unless noted otherwise, all references in this prospectus to share and per share amounts reflect the Reverse Stock Split.

Concurrently with the execution and delivery of the Merger Agreement, and in order to provide Neurogene OpCo with additional capital for its development programs, Neurogene OpCo entered into a subscription agreement (the “Subscription Agreement”) with certain investors named therein (the “Investors”), pursuant to which, subject to the terms and conditions of the Subscription Agreement, immediately prior to the effective time of the Reverse Merger, Neurogene OpCo issued and sold, and the Investors purchased, 2,792,206 shares of Neurogene OpCo common stock and 1,811,739 pre-funded warrants, exercisable for 1,811,739 shares of Neurogene OpCo common stock, at a purchase price of approximately \$20.63 per share or \$20.63 per warrant, for an aggregate purchase price of approximately \$95.0 million (the “Pre-Closing Financing”).

### **Closing of Private Placement**

On November 5, 2024, we closed a private investment in public equity financing (the “November 2024 private placement”) in which we sold 1,835,000 shares of common stock at a price of \$50.00 per share and, in lieu of shares of common stock, pre-funded warrants to purchase up to an aggregate of 2,165,042 shares of common stock, at a purchase price of \$49.999 per pre-funded warrant, to certain institutional accredited investors. The pre-funded warrants are immediately exercisable until exercised in full at a price of \$0.001 per share of common stock. The aggregate gross proceeds to the Company totaled approximately \$200.0 million. Net proceeds, net of commissions and other offering expenses, are expected to total approximately \$189.5 million.

### **Background**

We were founded in 2018, and have devoted substantially all of our resources to conducting research and development activities (including with respect to the NGN-401 and NGN-101 programs) and undertaking preclinical studies, establishing our manufacturing facility, conducting clinical trials and the manufacturing of product used in our clinical trials and preclinical studies, business planning, developing and maintaining our intellectual property portfolio, hiring personnel, raising capital, and providing general and administrative support for these activities.

Since our inception, we have funded our operations primarily with outside capital (e.g., proceeds from the sale of preferred stock and common stock) and have raised aggregate net proceeds of approximately \$521.9 million from these private placements. However, we have incurred significant recurring losses, including a net loss of \$55.6 million and \$38.7 million for the nine months ended September 30, 2024 and 2023, respectively. In addition, as of September 30, 2024, we had an accumulated deficit of \$242.8 million and cash, cash equivalents and short term investments totaling \$139.0 million. In order to continue our operations, we must achieve profitable operations and/or obtain additional equity or debt financing. Until we achieve profitability, management plans to fund our operations and capital expenditures with cash on hand and the sale and issuance of securities. There can be no assurance that we will be successful in raising additional capital or that such capital, if available, will be on terms that are acceptable to us. If we are unable to raise sufficient additional capital, we may be compelled to consider

actions such as reducing the scope of our operations and planned capital expenditures or selling certain assets, including intellectual property assets.

Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on a variety of factors, including the timing, scope and results of our research and development activities. Management expects that our expenses and capital requirements will increase substantially in connection with our ongoing activities as we:

- advance the NGN-401 program through clinical development;
- advance discovery programs from preclinical development into and through clinical development;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- establish sales, marketing and distribution infrastructure to commercialize any approved product candidates;
- establish a commercialization infrastructure and scale up internal and external manufacturing and distribution capabilities to commercialize any product candidates for which we may obtain regulatory approval;
- expand clinical, scientific, management and administrative teams;
- maintain, expand, protect and enforce our intellectual property portfolio, including patents, trade secrets and know-how;
- implement operational, financial and management systems; and
- incur additional legal, accounting and other expenses related to operating as a public company.

We do not have any products approved for commercial sale and have not generated any commercial revenue from product sales. Our ability to generate product revenue sufficient to achieve and maintain profitability will depend upon the successful development and eventual commercialization of one or more of our product candidates, which we expect, if it ever occurs, will take many years. We expect to spend a significant amount in development and marketing costs prior to such time. We will therefore require substantial additional capital to develop our product candidates and support our continuing operations. We may never succeed in achieving regulatory and marketing approval for our product candidates. We may obtain unexpected results from our preclinical and clinical trials. In November 2024, we announced that the Company does not expect to move forward with the NGN-101 CLN5 Batten disease gene therapy program at this time. Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that the application met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. We are currently evaluating options for the program. We may in the future elect to discontinue, delay, or modify additional preclinical and clinical trials of our other product candidates. A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. Accordingly, until such time that we can generate a sufficient amount of revenue from product sales or other sources, if ever, management expects to finance our operations through private or public equity or debt financings, loans or other capital sources, which could include income from collaborations, partnerships or other marketing, distribution, licensing or other strategic arrangements with third parties, or from grants. However, we may be unable to raise additional capital from these sources on favorable terms, or at all. Our failure to obtain sufficient capital on acceptable terms when needed could have a material adverse effect on our business, results of operations or financial condition, including requiring us to delay, reduce or curtail our research, product development or future commercialization efforts. We may also be required to license rights to product candidates at an earlier stage of development or on less favorable terms than we would otherwise choose. Our management cannot provide assurance that we will ever generate positive cash flow from operating activities. See “*Liquidity and Capital Resources.*”

In December 2020, we entered into the Master Research Collaboration (“MCA”) with the University of Edinburgh (the “University of Edinburgh”) to support our pipeline development and expansion, and to accelerate scientific innovation to continue to improve upon conventional gene therapy. In November 2023, the collaboration agreement was amended and extended through December 2026. The University of Edinburgh has a vibrant community of over 500 neuroscience researchers and is widely recognized as a preeminent center for neuroscience research, especially in areas of neurodegeneration and in neurodevelopmental disorders, such as Rett syndrome. For example, researchers currently in neuroscience centers at the University of Edinburgh conducted the seminal preclinical work for Rett syndrome, including discovery of the MeCP2 protein, its function as a transcriptional repressor, developing the first and most widely adopted animal model of Rett syndrome, demonstrating for the first time the reversibility of phenotypes in any neurodevelopmental disorder as well as the first ever preclinical gene therapy efforts in Rett syndrome. Under the terms of the agreement, we have the option to in-license product candidates from Dr. Stuart Cobb’s laboratory, where he has a dual appointment as a Professor in Translational Neuroscience at the Patrick Wild Centre and the Centre for Discovery Brain Sciences and serves as our Chief Scientific Officer. Dr. Cobb may be entitled to receive in the future a percentage of certain license-related payments from Neurogene to the University of Edinburgh in accordance with the University of Edinburgh’s standard policies for professor inventors.

### **Impact of Global Economic Events**

Uncertainty in the global economy presents significant risks to our business. We are subject to continuing risks and uncertainties in connection with the current macroeconomic environment, including high inflation, changes in interest rates, changes in foreign currency exchange rates, recent bank failures, proposed or adopted federal U.S. legislation seeking to limit the provision of services in our sector by certain non-U.S. entities, geopolitical factors, including the ongoing conflicts between Russia and Ukraine and in Israel and the surrounding areas and the responses thereto, the impacts of climate change, and supply chain disruptions. While management is closely monitoring the impact of the current macroeconomic conditions on all aspects of our business, including the impacts on our participants in our Phase 1/2 clinical trial, employees, suppliers, vendors and business partners, the ultimate extent of the impact on our business remains highly uncertain and will depend on future developments and factors that continue to evolve. Most of these developments and factors are outside of our control and could exist for an extended period of time. Management will continue to evaluate the nature and extent of the potential impacts to our business, results of operations, liquidity and capital resources. For additional information, see the section entitled “Risk Factors.”

### **Components of Results of Operations**

#### ***Revenue***

We have no products approved for sale and have never generated any revenue from product sales.

We have generated licensing revenue from the recognition of upfront payments received under agreements with third parties for the disposition of legacy Neoleukin assets (the “December 2023 CVR Licensing Agreement” and the “April 2024 CVR Licensing Agreement”) that are related to the legacy Neoleukin business as part of the reverse merger. See Note 9, *Commitments and Contingencies*, for additional details regarding these licensing agreements.

#### ***Operating Expenses***

##### ***Research and Development Expenses***

Research and development expenses consist primarily of costs incurred in connection with the discovery and development of our product candidates. We expense research and development costs as incurred, including:

- expenses incurred to conduct the necessary discovery-stage laboratory work, preclinical studies and clinical trials required to obtain regulatory approval;
- acquired licenses and intellectual property that are accounted for as asset acquisitions and have no alternative future use;

- personnel expenses, including salaries, benefits and stock-based compensation expense for our employees engaged in research and development functions;
- costs of funding research performed by third parties, including pursuant to agreements with clinical research organizations (“CROs”) that conduct our clinical trials, as well as investigative sites, consultants and CROs that conduct our preclinical and nonclinical studies;
- expenses incurred under agreements with our third-party contract development and manufacturing organizations (“CDMOs”), as well as internal manufacturing scale-up expenses, including the cost of acquiring and manufacturing preclinical study and clinical trial materials;
- fees paid to consultants who assist with research and development activities;
- expenses related to regulatory activities, including filing fees paid to regulatory agencies; and
- allocated expenses for facility costs, including rent, utilities, depreciation and maintenance.

Before a product receives regulatory approval, we record upfront and milestone payments to third parties under licensing arrangements as expense, provided that there is no alternative future use of the rights in other research and development projects.

Non-refundable prepayments for research and development costs that are paid in advance of performance are capitalized as a prepaid expense and amortized over the service period as the services are provided. Costs for certain development activities, such as outside research programs funded by us, are recognized based on an evaluation of the progress to completion of specific tasks with respect to their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the financial statements as prepaid or accrued research and development expense as applicable.

We track outsourced development costs and other external research and development costs to specific product candidates on a program-by-program basis, including fees paid to CROs, CDMOs and research laboratories in connection with our preclinical development, process development, and clinical development activities. We also incur personnel and other operating expenses for research and development programs, which are presented in aggregate.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect our research and development expenses to increase significantly over the next several years as we increase personnel costs, including stock-based compensation, conduct clinical trials, including later-stage clinical trials for current and future product candidates, and prepare regulatory filings for our product candidates.

#### *General and Administrative Expenses*

General and administrative expenses consist primarily of personnel expenses, including salaries, benefits and stock-based compensation expense, for employees and consultants in executive, finance and accounting, legal, operations support, information technology and human resource functions. General and administrative expenses also include corporate facility costs not otherwise included in research and development expense, including rent, utilities, depreciation and maintenance, as well as legal fees related to intellectual property and corporate matters and fees for accounting and consulting services.

We expect that our general and administrative expense will increase in the future to support our continued research and development activities, potential commercialization efforts and increased costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, legal support and accountants, among other expenses. Additionally, we anticipate increased costs associated with being a public company, including expenses related to services associated with maintaining compliance with the requirements of Nasdaq and the SEC, insurance and investor relations costs. If any of our current or future product candidates obtains U.S. regulatory approval, we expect that we would incur significantly

increased expenses associated with building a sales and marketing team, as well as an expanded regulatory and compliance function.

#### *Interest Income*

Interest income consists primarily of interest earned on our cash equivalents and short term investments. We expect our interest income to fluctuate depending on interest rates and the amount of cash that is invested.

#### *Income Taxes*

We assess our income tax positions and record tax benefits based upon management’s evaluation of the facts, circumstances, and information available at the reporting date. For those tax positions where it is more likely than not that a tax benefit will be sustained, we record the amount of tax benefit with a greater than 50 percent likelihood of being realized upon ultimate settlement with a taxing authority having full knowledge of all relevant information. For those income tax positions for which it is not more likely than not that a tax benefit will be sustained, no tax benefit is recognized in the financial statements.

Since inception, we have not recorded any income tax benefits for net operating losses (“NOLs”) or for our research and development tax credits, as we believe, based upon the weight of available evidence, that it is more likely than not that all of our NOLs and tax credits will not be realized. Accordingly, we have established a valuation allowance against such deferred tax assets for all periods since inception.

As of December 31, 2023, we had federal and state NOL carryforwards in the amount of \$277.9 million and \$35.1 million, respectively, which may be available to offset future taxable income. The state NOL carryforwards will begin to expire in 2038, unless previously utilized. Most federal NOL carryforwards were generated subsequent to January 1, 2018, and therefore are able to be carried forward indefinitely. As of December 31, 2023, we also had federal research tax credit and federal orphan drug tax credit carryforwards of \$7.5 million and \$2.2 million, respectively, which may be used to offset future tax liabilities. These tax and orphan drug credit carryforwards begin to expire in 2039 and 2042, respectively, unless previously utilized.

### **Results of Operations**

#### *Comparison of the Years Ended December 31, 2023 and 2022*

The following table summarizes our results of operations for the periods indicated:

(in thousands)	Year Ended December 31,		
	2023	2022	Change
Operating expenses:			
Research and development	\$ 44,394	\$ 47,505	\$ (3,111)
General and administrative	11,189	9,012	2,177
Total operating expenses	55,583	56,517	(934)
Loss from operations	(55,583)	(56,517)	934
Other income (expense):			
Interest income	2,951	1,337	1,614
Interest expense	(12)	(2)	(10)
Bargain purchase gain	16,355	—	16,355
Other expense	(28)	(7)	(21)
Net loss	\$ (36,317)	\$ (55,189)	\$ 18,872

### Research and Development Expenses

The following table summarizes our research and development expenses for the periods indicated:

(in thousands)	Year Ended December 31,		
	2023	2022	Change
Program specific expenses:			
Rett syndrome	\$ 5,957	\$ 4,609	\$ 1,348
Batten disease	5,507	5,576	(69)
Early Discovery	2,945	1,327	1,618
Discontinued Programs	351	3,861	(3,510)
Unallocated internal expenses:			
Personnel-related	15,328	16,152	(824)
Share-based compensation	897	732	165
Manufacturing	10,502	12,231	(1,729)
Other	2,907	3,017	(110)
Total research and development expenses	\$ 44,394	\$ 47,505	\$ (3,111)

Research and development expenses were \$44.4 million for the year ended December 31, 2023, as compared to \$47.5 million for the year ended December 31, 2022, a decrease of \$3.1 million.

Expenses related to the Rett syndrome program increased primarily due to a \$4.2 million increase in clinical trial costs related to the Phase 1/2 clinical trial of NGN-401, offset by a \$2.6 million decrease in preclinical costs. The decrease in expenses related to the Batten disease program was driven primarily by a decrease of \$0.1 million in clinical development expenses. The increase in Early Discovery expenses was driven primarily by a \$1.5 million increase in preclinical costs. Discontinued Programs expense decreased primarily due to a \$1.3 million decrease in preclinical costs, a \$1.1 million decrease in chemistry, manufacturing and controls costs, a \$0.5 million decrease in clinical trial costs, and \$0.5 million decrease in milestone payments. Expenses for Discontinued Programs were substantially complete by year end 2023 and are not expected to contribute meaningfully in 2024.

The \$2.5 million decrease in unallocated internal expenses was primarily driven by a \$1.7 million decrease in manufacturing expenses due to lower raw material expenses and a \$0.8 million decrease in personnel-related expenses primarily driven by \$0.7 million in employee retention tax credits received in 2023.

### General and Administrative Expenses

General and administrative expenses were \$11.2 million for the year ended December 31, 2023, as compared to \$9.0 million for the year ended December 31, 2022, an increase of \$2.2 million. The increase was attributable to: (i) a business email compromise attack by a third party, which resulted in the diversion of payments totaling approximately \$0.9 million to fraudulent bank accounts that was partially offset by the recovery of approximately \$0.5 million of the diverted funds; (ii) increases in personnel costs totaling \$0.7 million, (iii) increases in professional and consulting fees of approximately \$0.6 million resulting from the merger, and (iv) increases in insurance and information technology costs of approximately \$0.4 million. Subsequent to December 31, 2023, we have recovered approximately an additional \$0.2 million of the funds diverted in our email compromise attack.

### Interest Income

Interest income increased by \$1.7 million for the year ended December 31, 2023, as compared to the year ended December 31, 2022. The increase was due to a significant rise in interest rates from 2022 to 2023, which was partially offset by a decrease in the average amount of our cash, cash equivalents and investments during the same time period.

### Bargain Purchase Gain

We recorded a bargain purchase gain of approximately \$16.4 million in connection with the Closing. The fair value of Neoleukin's net assets acquired at the Closing exceeded the total consideration transferred after the fair value allocation by approximately \$17.6 million. \$1.3 million of the difference was recorded as a contingent consideration liability for payments that are probable and estimable under the CVR Agreement, and the remaining \$16.4 million was recognized as a bargain purchase gain.

### Comparison of the Three and Nine Months Ended September 30, 2024 and 2023

The following table summarizes our results of operations for the periods indicated (in thousands):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2024	2023	Change	2024	2023	Change
Revenue under licensing agreements	\$ —	\$ —	\$ —	\$ 925	\$ —	\$ 925
Operating expenses:						
Research and development expenses	16,285	11,606	4,679	45,570	32,210	13,360
General and administrative expenses	5,895	3,613	2,282	16,448	8,640	7,808
Total operating expenses	22,180	15,219	6,961	62,018	40,850	21,168
Loss from operations	(22,180)	(15,219)	(6,961)	(61,093)	(40,850)	(20,243)
Other income (expense):						
Interest income	1,842	650	1,192	6,197	2,170	4,027
Interest expense	(3)	(4)	1	(10)	(9)	(1)
Other income	143	—	143	430	—	430
Other expense	(19)	(4)	(15)	(1,154)	(11)	(1,143)
Net loss	\$ (20,217)	\$ (14,577)	\$ (5,640)	\$ (55,630)	\$ (38,700)	\$ (16,930)

### Revenue

The Company did not generate any revenue for either the three months ended September 30, 2024 or for the three months ended September 30, 2023.

Licensing revenue was \$0.9 million for the nine months ended September 30, 2024, as compared to \$0 for the nine months ended September 30, 2023. We generated licensing revenue from the recognition of upfront payments received under the December 2023 CVR Licensing Agreement and the April 2024 CVR Licensing Agreement. Please see the section below entitled *Other Expenses* for a discussion on the related CVR liabilities.

### Research and Development Expenses

The following table summarizes our research and development expenses for the periods indicated (in thousands):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2024	2023	Change	2024	2023	Change
<b>Program specific expenses:</b>						
Rett syndrome	\$ 3,444	\$ 1,665	\$ 1,779	\$ 8,261	\$ 3,917	\$ 4,344
Batten disease	1,832	1,391	441	4,756	3,944	812
Early discovery	1,329	857	472	4,500	1,743	2,757
<b>Unallocated internal expenses:</b>						
Personnel-related	4,512	4,103	409	13,787	11,481	2,306
Share-based compensation	1,272	242	1,030	3,211	657	2,554
Manufacturing	3,220	2,605	615	8,840	7,906	934
Other	676	743	(67)	2,215	2,562	(347)
<b>Total research and development expenses</b>	<b>\$ 16,285</b>	<b>\$ 11,606</b>	<b>\$ 4,679</b>	<b>\$ 45,570</b>	<b>\$ 32,210</b>	<b>\$ 13,360</b>

Research and development expenses were \$16.3 million for the three months ended September 30, 2024, as compared to \$11.6 million for the three months ended September 30, 2023.

Expenses related to the Rett syndrome program increased primarily due to a \$1.5 million increase in clinical trial costs for the Phase 1/2 clinical trial of NGN-401. The increase in expenses related to the Batten disease program was primarily driven by a \$0.7 million increase in clinical trial costs for the Phase 1/2 clinical trial of NGN-101 and a \$0.3 million increase in clinical development costs, offset by a reduction of \$0.4 million in chemistry, manufacturing and control costs. The increase in expenses related to the Early discovery program was primarily driven by a \$0.4 million increase in preclinical costs.

The increase in unallocated internal expenses was driven primarily by higher salaries, benefits, and share-based compensation costs due to an increase in research and development headcount, as well as an increase in laboratory consumables expense related to chemistry, manufacturing and controls.

Research and development expenses were \$45.6 million for the nine months ended September 30, 2024, as compared to \$32.2 million for the nine months ended September 30, 2023.

Expenses related to the Rett syndrome program increased primarily due to a \$4.1 million increase in clinical trial costs for the Phase 1/2 clinical trial of NGN-401. The increase in expenses related to the Batten disease program was primarily driven by a \$0.6 million increase in clinical trial costs for the Phase 1/2 clinical trial of NGN-101, and \$0.5 million increase in clinical development costs, offset by a reduction of \$0.4 million in chemistry, manufacturing and control costs. The increase in expenses related to the Early discovery program was primarily driven by a \$2.6 million increase in preclinical costs.

The increase in unallocated internal expenses was driven primarily by higher salaries, benefits, and share-based compensation costs due to an increase in research and development headcount, as well as an increase in laboratory consumables expense related to chemistry, manufacturing and controls.

We expect that our research and development expenses will continue to increase for the foreseeable future as we advance our programs and product candidates into and through clinical development and, as we continue to develop additional product candidates, build our manufacturing capabilities and develop our EXACT technology.



### *General and Administrative Expenses*

General and administrative expenses were \$5.9 million for the three months ended September 30, 2024, as compared to \$3.6 million for the three months ended September 30, 2023. The increase was primarily attributable to: (i) approximately \$1.6 million in employee-related expenses, including an increase of \$1.1 million in stock-based compensation expense, driven by an increase in headcount to support our transition into a public company, (ii) approximately \$0.3 million in professional services and consulting fees, (iii) approximately \$0.6 million related to rent associated with the Company's assumption of legacy Neoleukin facilities (the Eastlake Lease and Blaine Lease), and (iv) \$0.6 million related to corporate related expenses and market research costs. This was partially offset by a business email compromise attack by a third party in the prior period. This resulted in the diversion of payments totaling approximately \$0.9 million to a fraudulent bank account. Subsequent to September 30, 2023, the Company has recovered approximately \$0.8 million of the diverted funds.

General and administrative expenses were \$16.4 million for the nine months ended September 30, 2024, as compared to \$8.6 million for the nine months ended September 30, 2023. The increase was primarily attributable to: (i) approximately \$3.8 million in employee-related expenses, including an increase of \$2.2 million in stock-based compensation expense, driven by an increase in headcount to support our transition into a public company, (ii) approximately \$1.8 million in professional services and consulting fees, (iii) approximately \$1.7 million related to rent associated with the Company's assumption of legacy Neoleukin facilities (the Eastlake Lease and Blaine Lease), and (iv) \$1.4 million related to corporate related expenses and market research costs. This was partially offset by a business email compromise attack by a third party in the prior period. This resulted in the diversion of payments totaling approximately \$0.9 million to a fraudulent bank account. Subsequent to September 30, 2023, the Company has recovered approximately \$0.8 million of the diverted funds.

We anticipate that our general and administrative expenses will increase in the future to support increased research and development activities.

### *Interest Income*

Interest income increased by \$1.2 million for the three months ended September 30, 2024, as compared to the three months ended September 30, 2023. The increase was primarily due to a significant increase in the amount of Neurogene's cash balances.

Interest income increased by \$4.0 million for the nine months ended September 30, 2024, as compared to the nine months ended September 30, 2023. The increase was primarily due to a significant increase in the amount of Neurogene's cash balances.

### *Other Income*

Other income increased by \$0.1 million for the three months ended September 30, 2024, as compared to the three months ended September 30, 2023. The increase was primarily due to sublease income associated with the Eastlake Lease assumed after the Reverse Merger closed.

Other income increased by \$0.4 million for the nine months ended September 30, 2024, as compared to the nine months ended September 30, 2023. The increase was primarily due to sublease income associated with the Eastlake Lease assumed after the Reverse Merger closed.

### *Other Expenses*

Other expenses were insignificant for the three months ended September 30, 2024 and for the three months ended September 30, 2023.

Other expenses increased by \$1.1 million for the nine months ended September 30, 2024, as compared to the nine months ended September 30, 2023. The increase was primarily due to (1) the accrual of contingent consideration liabilities related to the Intellectual Property CVR (as defined in Note 9, *Commitments and Contingencies*, in the notes to the financial statements included in Part I, Item 1 of this Quarterly Report on this prospectus in connection with the December 2023 Licensing Agreement and the April 2024 Licensing Agreement and

(2) the accrual of a contingent consideration liability related to the Sales Tax CVR for an anticipated sales tax refund from Washington state.

## **Liquidity and Capital Resources**

### ***Sources of Liquidity***

Since inception, we have not generated any revenue from product sales and have incurred significant operating losses and negative cash flows from our operations. We expect to continue to incur significant expenses and operating losses for the foreseeable future as we advance the clinical development of our product candidates. We expect that our research and development and general and administrative costs will continue to increase significantly, including in connection with conducting clinical trials and manufacturing for our product candidates to support commercialization and providing general and administrative support for our operations, including the costs associated with operating as a public company. As a result, we will need additional capital to fund our operations, which we may obtain from additional equity or debt financings, collaborations, licensing arrangements or other sources. We believe that our existing capital resources will be sufficient to fund our operations through at least 12 months following the filing date of this Form 10-Q. See the section entitled “Risk Factors” for additional risks associated with our substantial capital requirements.

As of September 30, 2024, we had cash, cash equivalents and short term investments totaling \$139.0 million. Since inception and through the issuance of these financial statements, we have funded our operations primarily through private placements of convertible preferred stock and common stock for net proceeds of approximately \$521.9 million, including the amount raised in the November 2024 Private Placement.

### ***Future Capital Requirements***

In order to complete the development of our product candidates and to build the sales, marketing and distribution infrastructure that management believes will be necessary to commercialize product candidates, if approved, we will require substantial additional capital. Accordingly, until such time as we can generate a sufficient amount of revenue from product sales or other sources, if ever, management expects to seek to raise any necessary additional capital through private or public equity or debt financings, loans or other capital sources, which could include income from collaborations, partnerships or other marketing, distribution, licensing or other strategic arrangements with third parties, or from grants. To the extent that we raise additional capital through equity financings or convertible debt securities, the ownership interest of our stockholders will be or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing and equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, including restricting our operations and limiting our ability to incur liens, issue additional debt, pay dividends, repurchase our own common stock, make certain investments or engage in merger, consolidation, licensing, or asset sale transactions. If we raise capital through collaborations, partnerships, and other similar arrangements with third parties, we may be required to grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. We may be unable to raise additional capital from these sources on favorable terms, or at all. Our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions and the recent disruptions to, and volatility in, the credit and financial markets in the United States and worldwide resulting from macroeconomic conditions, geopolitical instability, government regulation and otherwise. The failure to obtain sufficient capital on acceptable terms when needed could have a material adverse effect on our business, results of operations or financial condition, including by requiring us to delay, reduce or curtail our research, product development or future commercialization efforts. We may also be required to license rights to product candidates at an earlier stage of development or on less favorable terms than we would otherwise choose. Management cannot provide assurance that we will ever generate positive cash flow from operating activities.

In order to continue our operations, we must achieve profitable operations and/or obtain additional equity or debt financing. Until we achieve profitability, management plans to fund our operations and capital expenditures with cash on hand and the sale and issuance of securities. We may not be successful in raising additional capital and such capital, if available, may not be on terms that are acceptable to us.

We have incurred, and expect to continue to incur, additional costs associated with operating as a public company. In addition, we anticipate that we will need substantial additional funding in connection with our continuing operations. Management bases its projections of operating capital requirements on our current operating plan, which includes several assumptions that may prove to be incorrect, and we may use all of our available capital resources sooner than management expects.

Because of the numerous risks and uncertainties associated with research, development and commercialization of product candidates, we are unable to estimate the exact amount and timing of our capital requirements. Our future funding requirements will depend on many factors, including:

- the scope, timing, progress, results, and costs of researching and developing genetic medicines, and conducting larger and later-stage clinical trials;
- the scope, timing, progress, results, and costs of researching and developing other product candidates that we may pursue;
- the costs, timing, and outcome of regulatory review of our product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, and distribution, for any of our product candidates for which we receive marketing approval;
- the costs of manufacturing commercial-grade products and sufficient inventory to support commercial launch;
- the revenue, if any, received from commercial sale of our products, should any of our product candidates receive marketing approval;
- the cost and timing of attracting, hiring, and retaining skilled personnel to support our operations and continued growth;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- Our ability to establish, maintain, and derive value from collaborations, partnerships or other marketing, distribution, licensing, or other strategic arrangements with third parties on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies, if any; and
- the costs associated with operating as a public company.

A change in the outcome of any of these or other factors with respect to the development of any of our product candidates could significantly change the costs and timing associated with the development of that product candidate. Furthermore, our operating plans may change in the future, and we may need additional capital to meet the capital requirements associated with such operating plans.

### ***Cash Flows***

The following table summarizes our cash flows for the annual periods indicated (in thousands):

	Year Ended December 31,	
	2023	2022
Net cash used in operating activities	\$ (51,422)	\$ (52,824)
Net cash provided by (used) in investing activities	25,637	(2,230)
Net cash provided by financing activities	92,482	66,531
Net increase in cash and cash equivalents	\$ 66,697	\$ 11,477

*Cash Flows from Operating Activities*

For the year ended December 31, 2023, we used \$51.4 million of cash in operating activities. Cash used in operating activities reflected our net loss of \$36.3 million, a \$4.1 million net increase in our operating assets and liabilities, and noncash charges of \$11.1 million. The non-cash charges consisted of a bargain purchase gain in connection with the merger of \$16.4 million, which was partially offset by \$3.3 million in depreciation, \$1.4 million in stock-based compensation and \$0.7 million in non-cash operating lease expense. The primary use of cash was to fund our operations related to the development of our product candidates.

For the year ended December 31, 2022, we used \$52.8 million of cash in operating activities. Cash used in operating activities reflected our net loss of \$55.2 million and a \$2.7 million net increase in our operating assets and liabilities, and was partially offset by noncash charges of \$5.1 million, which consisted of \$3.2 million in depreciation, \$1.3 million in stock-based compensation and \$0.6 million in non-cash operating lease expense. The primary use of cash was to fund our operations related to the development of our product candidates.

*Cash Flows from Investing Activities*

For the year ended December 31, 2023, net cash flows provided by investing activities consisted of cash acquired from the merger of \$22.2 million and maturities on short-term investments of \$5.0 million, which were partially offset by merger transaction costs paid of \$1.3 million and purchases of property and equipment of \$0.3 million.

For the year ended December 31, 2022, net cash flows used in investing activities consisted of purchases of property and equipment of \$2.2 million.

*Cash Flows from Financing Activities*

For the year ended December 31, 2023, net cash flows provided by financing activities consisted of net proceeds from the issuance of common stock and pre-funded warrants in the Pre-Closing Financing of \$92.3 million and proceeds from the issuance of common stock upon the exercise of options of \$0.2 million.

For the year ended December 31, 2022, net cash flows provided by financing activities consisted of proceeds from the issuance of Series B convertible preferred stock of \$66.5 million and proceeds from the issuance of Series A common stock upon the exercise of options of \$0.1 million.

The following table summarizes our cash flows for the interim periods indicated (in thousands):

	Nine Months Ended September 30,	
	2024	2023
Net cash used in operating activities	\$ (53,107)	\$ (34,450)
Net cash used in investing activities	(22,645)	(193)
Net cash used in financing activities	(5,994)	(1,815)
Net decrease in cash, cash equivalents and restricted cash	<u>\$ (81,746)</u>	<u>\$ (36,458)</u>

*Cash Flows from Operating Activities*

For the nine months ended September 30, 2024, we used \$53.1 million of cash in operating activities. Cash used in operating activities reflected our net loss of \$55.6 million, a \$5.1 million net decrease in our operating assets and liabilities and noncash charges of \$7.6 million, which consisted primarily of \$5.8 million of stock-based compensation, \$2.4 million in depreciation and \$0.5 million in non-cash operating lease expense, partially offset by \$1.5 million in accretion on the held-to-maturity investments. The primary use of cash was to fund our operations related to the development of our product candidates, costs associated with the Reverse Merger and the Pre-Closing Financing, and related severance and retention payments to former Neoleukin employees.

For the nine months ended September 30, 2023, we used \$34.5 million of cash in operating activities. Cash used in operating activities reflected our net loss of \$38.7 million and a \$0.2 million net increase in our operating assets and liabilities, offset by noncash charges of \$4.0 million, which consisted primarily of \$2.4 million in depreciation, \$1.1 million in stock-based compensation and \$0.5 million in non-cash operating lease expense. The primary use of cash was to fund our operations related to the development of our product candidates.

*Cash Flows from Investing Activities*

For the nine months ended September 30, 2024, net cash flows used in investing activities consisted of purchases of investments of \$80.2 million and purchases of property and equipment of \$0.7 million, partially offset by purchases of investments of \$58.3 million.

For the nine months ended September 30, 2023, net cash flows used in investing activities consisted of purchases of property and equipment of \$0.2 million.

*Cash Flows from Financing Activities*

For the nine months ended September 30, 2024, net cash flows used in financing activities consisted of \$4.3 million in offering costs paid in connection with the Pre-Closing Financing and \$2.9 million in transaction costs related to the Reverse Merger, partially offset by proceeds of \$1.2 million from the exercise of stock options.

For the nine months ended September 30, 2023, net cash flows provided by financing activities consisted of \$2.0 million in offering costs paid in connection with the Pre-Closing Financing, partially offset by proceeds of \$0.2 million from the exercise of stock options.

**Contractual Obligations and Commitments**

***Lease Obligations***

*New York Headquarters Lease*

We sub-lease approximately 6,000 square feet of office space for our corporate headquarters in New York, New York, with a term expiring in June 2026.

*Houston Lease*

We lease 42,342 square feet for a manufacturing facility in Houston, Texas. The lease expires in August 2029. We have the option to renew the lease term for two additional five-year terms. The renewal periods were not included in the lease term for purposes of determining the lease liability or right-of-use asset.

*Blaine Lease in Seattle*

We lease approximately 33,300 square feet of office space in Seattle, Washington that was previously used by Neoleukin for offices, a laboratory for research and development, and related uses (the "Blaine Lease"). The lease expires on February 1, 2029, with the option to extend the lease for two five-year terms. The renewal periods were not included in the lease term for purposes of determining the lease liability.

*Eastlake Lease in Seattle*

We lease approximately 6,272 square feet of office space in Seattle, Washington, that was previously used by Neoleukin for additional office and laboratory space for research and development and related uses (the "Eastlake Lease"). We also assumed the existing agreement to sublease the Eastlake Lease to an unrelated third party ("Eastlake Sublease"). Pursuant to the terms of the Eastlake Sublease, we are entitled to receive a total of approximately \$1.6 million in lease payments. The term of the sublease is through September 30, 2026.

### **Lease CVR**

Each contingent value right (“CVR”) distributed pursuant to the CVR Agreement, dated December 18, 2023, by and between the Company and the Rights Agent (the “CVR Agreement”) contains the contractual right to receive certain net savings, if any, realized by June 30, 2029 in connection with certain legacy lease obligations related to our business prior to the Reverse Merger (the “Lease CVR”). As of September 30, 2024, approximately \$1.2 million was recorded as a component of the contingent value rights liability arising from the Lease CVR on our condensed consolidated balance sheet. The commitment relates to Neoleukin’s sublease agreement, effective October 31, 2023, for one of its properties with an unrelated third party for the remainder of the lease term. For more information on the Lease CVR, see Note 9, *Commitments and Contingencies—Lease CVR*, in the notes to the financial statements included in this prospectus.

### **Intellectual Property CVR**

The December 2023 CVR Licensing Agreement and April 2024 CVR Agreement collectively account for the total Intellectual Property CVR. As of September 30, 2024, approximately \$0.1 million is recorded within the contingent value rights liability as an offset arising from the Intellectual Property CVR on the Company’s condensed consolidated balance sheet due to deductions permitted under the Merger Agreement. For more information on the Intellectual Property CVR, see Note 9, *Commitments and Contingencies—Intellectual Property CVR*, in the notes to the financial statements included in Part I, Item 1 of this prospectus.

### **Sales Tax CVR**

In accordance with the terms of the Sales Tax CVR within the CVR Agreement, we accrued a contingent consideration liability on our condensed consolidated balance sheet. The terms of the CVR Agreement include that CVR holders are eligible to receive certain net proceeds derived from an anticipated sales tax refund from Washington state relating to tax returns filed by Neoleukin prior to Closing. As of September 30, 2024, the liability in the condensed consolidated financial statements arising from the Sales Tax CVR was \$0.3 million. For more information on the Sales Tax CVR, see Note 9, *Commitments and Contingencies—Sales Tax CVR*, in the notes to the financial statements included in Part I, Item 1 of this prospectus.

The following tables summarize our contractual obligations and commitments as of September 30, 2024 (in thousands):

<b>Maturity of operating lease liabilities</b>	
2024 (remaining)	\$ 982
2025	3,987
2026	3,695
2027	3,239
2028	3,294
2029	613
Total lease payments	<u>\$ 15,810</u>
<b>Maturity of finance lease liabilities</b>	
2024 (remaining)	\$ 15
2025	60
2026	21
2027	6
Total lease payments	<u>\$ 102</u>

	September 30, 2024		December 31, 2023	
	Current	Non-Current	Current	Non-Current
Lease CVR	\$ 436	\$ 717	\$ 281	\$ 1,006
Intellectual Property CVR	—	(71)	—	—
Sales Tax CVR	332	—	—	—
Total CVR liability	\$ 768	\$ 646	\$ 281	\$ 1,006

### **Research and Development and Manufacturing Agreements**

We enter into agreements with certain vendors for the provision of goods and services, which includes manufacturing services with contract development and manufacturing organizations and development and clinical trial services with CROs. These agreements may include certain provisions for purchase obligations and termination obligations that could require payments for the cancellation of committed purchase obligations or for early termination of the agreements. The amount of the cancellation or termination payments vary and are based on the timing of the cancellation or termination and the specific terms of the agreement. These obligations and commitments are not presented separately.

### **License and Collaboration Agreements**

#### *License Agreement with The University of North Carolina*

In May 2019, Neurogene entered into an Exclusive License Agreement with the University of North Carolina at Chapel Hill (“UNC”) to obtain an exclusive, worldwide, royalty bearing license, with the right to grant sublicenses under certain patents to make, use, or sell products covered by such patents for prevention or treatment of disease or medical or genetic conditions, including CLN5 Batten disease or other diseases from dysfunction of the CLN5 gene. Neurogene is obligated to pay UNC up to \$1.7 million in sales-related milestones for licensed products based on annual sales of the licensed product in excess of defined thresholds and low single-digit percentage royalties on net sales of licensed product for as long as there is a valid patent claim under the patent rights. Neurogene is also required to reimburse any patent expenses, as well as pay a nonrefundable annual maintenance fee which, when royalties become due and payable, will be creditable against such royalties. In November 2024, we announced that the Company does not expect to move forward with the NGN-101 CLN5 Batten disease gene therapy program at this time. Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. We are currently evaluating options for the program.

#### *License Agreement with The University of Edinburgh*

In December 2020, we entered into the MCA with University of Edinburgh. Under the MCA, Neurogene and the University of Edinburgh agreed to collaborate on certain research and development projects (“Projects”), and we agreed to provide funding for such Projects for a 40-month initial term, which term was extended in November 2023 for an additional 33 months and may be further extended by mutual agreement. In exchange for such funding, the University of Edinburgh granted us the option to exclusively license any intellectual property arising from such Projects. Under the MCA, we are obligated to pay semi-annual installment payments relating to funding of costs for personnel and lab consumables for the 40-month period. Either party may terminate the MCA for convenience upon 90 days’ notice. If we terminate the MCA, we would be responsible for all non-cancellable costs and commitments related to any particular Project and any and all funding costs for any person working on such Project.

In March 2022, we exercised our option through the collaboration under the MCA, and entered into a License Agreement (the “March 2022 Edinburgh License Agreement”) with University of Edinburgh, pursuant to which we licensed certain patents and know-how related to the EXACT technology and optimized *MECP2* cassettes on an exclusive basis. Under the March 2022 Edinburgh License Agreement, we obtained an exclusive, worldwide license to the licensed patents to develop, manufacture, supply, sell, and commercialize any products that utilize the licensed patents (the “Licensed Products”) in exchange for low single-digit percentage royalties on future commercial net

sales of the Licensed Products. Royalties are payable on a Licensed Product-by-Licensed Product and country-by-country basis until the later of the expiration of the last licensed patent covering such Licensed Product in the country where the Licensed Product is sold, or, if no licensed patent exists or has expired in such country, then ten years from first commercial sale of such Licensed Product in such country (the “Royalty Term”). The term of the March 2022 Edinburgh License Agreement continues until the end of the Royalty Term and the expiration of all of the payment obligation thereunder. We may terminate the March 2022 Edinburgh License Agreement for convenience upon 90 days’ notice. In connection with the license, we are also obligated to pay the University of Edinburgh up to \$5.3 million in regulatory-related milestones and up to \$25.0 million in sales-related milestones based on annual net sales of Licensed Products in excess of defined thresholds.

*License Agreement with Virovek*

In September 2020, we entered into a Non-Exclusive License Agreement with Virovek, Inc., pursuant to which we have a license to use certain patents and know-how on a non-exclusive basis related to our baculovirus process in exchange for low single-digit percentage royalties on future commercial net sales of each product using the baculovirus process, development milestone payments of up to \$0.2 million in the aggregate, and a nonrefundable annual license fee. This agreement continues until the later of (a) the expiration of the last to expire patent right that covers the manufacture, use, offer for sale, sale, importation, export or supply of any licensed product, (b) ten years after the first commercial sale of any licensed product, or (c) the expiration of all regulatory or market exclusivities. We may terminate this agreement for convenience upon 60 days’ notice.

*License Agreement with Sigma-Aldrich Co*

In January 2023, we entered into a Non-Exclusive License Agreement with Sigma-Aldrich Co. LLC, pursuant to which we have a license to certain patents and know-how on a non-exclusive basis related to certain cell lines used in our baculovirus process in exchange for a small annual fee on a product-by-product basis, payable once the first product candidate enters the clinic. In addition, on a product-by-product basis, we are obligated to pay up to \$2.5 million in the aggregate for development-related milestones. This agreement remains in force for as long as we continue to possess and use the licensed technology. We may terminate this agreement for convenience upon 60 days’ notice.

*License Agreement with Stanford*

In August 2024, we entered into a Nonexclusive License Agreement with the Board of Trustees of Leland Stanford Junior University (the “Stanford License Agreement”) to license, on a non-exclusive basis, certain biological materials used in the manufacturing process of our product candidates, including NGN-401. Over the 10-year term of the Stanford License Agreement, we are obligated to pay up to \$0.5 million in licensing fees. We may terminate this agreement for convenience upon 30 days’ notice.

**Off-Balance Sheet Arrangements**

We currently do not have, and did not have during the periods presented, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

**Critical Accounting Policies and Significant Judgments and Estimates**

Our financial statements are prepared in accordance with U.S. GAAP. The preparation of the financial statements and related disclosures requires management to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that management believes are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Management evaluates estimates and assumptions on a periodic basis. Our actual results may differ from these estimates.



While our significant accounting policies are described in more detail in Note 3 to the financial statements for the years ended December 31, 2023 and 2022, appearing elsewhere in this prospectus, management believes that the following accounting policies are critical to understanding our historical and future performance, as the policies relate to the more significant areas involving management's judgments and estimates used in the preparation of the financial statements.

### ***Research and Development Expenses***

Research and development expenses consist primarily of costs incurred in connection with the development of our product candidates. We expense research and development costs as incurred.

These costs include, but are not limited to, employee-related expenses, including salaries, benefits and travel of research and development personnel, facilities, supplies, rent, insurance, stock-based compensation, depreciation and external expenses incurred under agreements with contract research organizations and investigative sites that conduct preclinical and clinical studies and manufacture the drug product for our preclinical and clinical activities and other costs associated with preclinical activities.

Before a product receives regulatory approval, we record upfront and milestone payments to third parties under licensing arrangements as expense, provided that there is no alternative future use of the rights in other research and development projects.

We accrue expenses for preclinical studies and clinical trial activities performed by our vendors based upon estimates of the proportion of work completed. We determine the estimates by reviewing contracts, vendor agreements and purchase orders, and through discussions with our internal clinical personnel and external service providers as to the progress or stage of completion of trials or services and the agreed-upon fee to be paid for such services. However, actual costs and timing of clinical trials are highly uncertain, subject to risks and may change depending upon a number of factors, including our clinical development plan. There can be judgment involved in measuring the research and development expenses to be recognized in a particular period. In some cases, expense is recorded using an underlying assumption of the progress to completion of specific activities. For example, costs may be recognized based on the passage of time for activities that span reporting periods. If the provision of services is not linear then this assumption could impact the amount of expense recognized. The level of judgment varies based on the nature of the services being performed and the underlying support obtained. For some activities, such as for certain clinical trials, expense is recorded based on information obtained from vendors as an intermediary to those performing the underlying services, such as contract research organizations. These estimates are inherently more judgmental since the quality and availability of the underlying data may vary. We do not need to make significant estimates where costs incurred are supported by invoices or reports of costs incurred are obtained from a vendor that is directly performing the underlying services, such as a consultant or contract manufacturing organization.

We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known at that time. If the actual timing of the performance of services or the level of effort varies from the estimate, we will adjust the accrual accordingly. Nonrefundable advance payments for goods and services, including fees for clinical trial expenses, process development or manufacturing and distribution of clinical supplies that will be used in future research and development activities, are deferred and recognized as expense in the period that the related goods are consumed or services are performed.

In-process research and development ("IPR&D") that is acquired through licensing arrangements and accounted for as asset acquisitions are expensed immediately and within research and development expenses if the IPR&D has no alternative future use.

### ***Stock-Based Compensation***

We account for stock options granted to employees and non-employees at fair value, which is measured using Black-Scholes Option pricing model. The fair value measurement date for employee awards is generally the date of grant. We recognize stock-based compensation expense over the requisite service period of the individual grant, generally equal to the vesting period and use the straight-line method to recognize stock-based compensation.

Our policy is to account for forfeitures of stock-based awards when they occur in accordance with *ASC 718, Compensation—Stock Compensation*. We reverse compensation cost previously recognized, in the period the award is forfeited, for an award that is forfeited before completion of the requisite service period.

We utilize the Black-Scholes option-pricing model, which incorporates assumptions and estimates, to value these options. Estimates and assumptions impacting the fair value measurement include the fair value per share of the underlying stock issuable upon exercise of the options, life of the options, risk-free interest rate, expected dividend yield and expected volatility from peer public companies of the price of the underlying stock.

#### *Estimating the Fair Value of Common Stock*

Prior to the merger, we were required to estimate the fair value of the common stock underlying our stock-based awards when performing the fair value calculations using the Black-Scholes option pricing model. Because our common stock was not publicly traded, the fair value of the common stock underlying our stock options was determined on each grant date by our board of directors, with input from management, considering our most recently available third-party valuation of common shares. Following the merger, the fair value of our common stock is based on the closing stock price on the date of grant as reported on Nasdaq.

Prior to the merger, the third-party valuations of our common stock were performed using methodologies, approaches and assumptions consistent with the American Institute of Certified Public Accountants, *Audit and Accounting Practice Aid Series: Valuation of Privately Held Company Equity Securities Issued as Compensation*. In addition, our board of directors considered various objective and subjective factors to estimate the estimated fair value of our common stock, including:

- contemporaneous valuations of our common stock performed by independent third-party specialists;
- prices of our convertible preferred stock sold to outside investors in arm's length transactions, and the rights, preferences and privileges of our convertible preferred stock as compared to those of our common stock, including the liquidation preferences of our convertible preferred stock;
- estimated value of each security both outstanding and anticipated;
- anticipated capital structure, which will directly impact the value of the currently outstanding securities;
- actual results of operations and financial position;
- the status of our research and development efforts;
- the composition of, and changes to, our management team and board of directors;
- the lack of marketability and liquidity of our common stock as a private company;
- our stage of development and business strategy and the material risks related to our business and industry;
- general external market conditions affecting the life sciences and biotechnology industry sectors;
- U.S. and global economic conditions;
- the likelihood of achieving a liquidity event for the holders of our common stock, such as an initial public offering or a sale of our company, given prevailing market conditions; and
- the market value and volatility of comparable companies.

In determining the estimated fair value of our common stock, our board of directors considered the subjective factors discussed above in conjunction with the most recent valuations of our common stock that were prepared by a third-party. Our board of directors, with the assistance of third parties, determined valuations of Neurogene's common stock of \$20.24 and \$18.39 per share as of March 4, 2022 and January 13, 2023, respectively, and such

valuations by the board of directors were used for the purposes of determining the stock-based compensation expense.

**Recent Accounting Pronouncements**

See Note 3, *Recently Issued Accounting Standards*, in the Notes to Financial Statements included in Part II Item 8 of this Annual Report on Form 10-K.

**Smaller Reporting Company Status**

We are a “smaller reporting company” as defined under the Exchange Act. We may continue to be a smaller reporting company if either (i) the market value of our stock held by non-affiliates is less than \$250.0 million or (ii) our annual revenue is less than \$100.0 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than \$700.0 million. As a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and smaller reporting companies have reduced disclosure obligations regarding executive compensation.

**CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE**

None.

## QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

As a “smaller reporting company,” as defined by Rule 12b-2 of the Exchange Act, and pursuant to Item 305 of Regulation S-K we are not required to provide quantitative and qualitative disclosures about market risk.

### BUSINESS

#### Company Overview

##### Overview

Despite recent scientific advances in genetics, most neurological diseases, particularly those with devastating consequences to patients, are left untreated. Conventional gene therapy is an attractive potential treatment approach for only a limited number of monogenic diseases due to the challenges caused by the complex biology of neurological diseases and by inherent variable transgene uptake and expression. We are a clinical-stage biotechnology company committed to overcoming these limitations and turning today’s complex devastating neurological diseases into treatable conditions. By harnessing our proprietary transgene regulation technology, EXACT™ (Expression Attenuation via Construct Tuning), we are building a robust and differentiated product portfolio of genetic medicines for rare neurological diseases with high unmet need not otherwise addressable by conventional gene therapy. Our EXACT approach leverages key scientific breakthroughs, including gene transfer technology, microRNA-based genetic circuits, and adeno-associated virus (“AAV”) delivery, and is designed to deliver therapeutic levels of transgene to key areas of the brain that underlie neurological disease pathology.

In addition to NGN-401, we have also been pursuing a conventional gene therapy program in an ongoing Phase 1/2 clinical trial of NGN-101 for the treatment of CLN5 Batten disease. This patient population has a significant unmet need and experiences extensive neurological and physical impairment leading to blindness, loss of motor function and early mortality. Our Phase 1/2 clinical trial of NGN-101 was the first trial to assess the treatment of both neurodegenerative and ocular disease manifestations of Batten disease. In November 2024, we announced that the Company does not expect to move forward with the NGN-101 CLN5 Batten disease gene therapy program at this time. Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. We are currently evaluating options for the program.

We also established a fully operational current good manufacturing practices (“cGMP”) facility in Houston, Texas used to manufacture current and future product for research, toxicology and clinical studies. We believe that our in-house manufacturing capabilities better enable control of product quality and development timelines, strategic pipeline and financial flexibility, and clinical-to-commercial continuity.

##### Completion of the Reverse Merger and Pre-Closing Financing

On December 18, 2023, we completed our business combination with Neurogene OpCo (the “Closing”) in accordance with the terms of the Agreement and Plan of Merger, dated as of July 17, 2023 (the “Merger Agreement”), by and among the Company, Project North Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of the Company (“Merger Sub”), and Neurogene OpCo, pursuant to which, among other matters, Merger Sub merged with and into Neurogene OpCo, with Neurogene OpCo surviving as a wholly owned subsidiary of the Company (the “Reverse Merger”). In connection with the completion of the Reverse Merger, the Company changed its name from “Neoleukin Therapeutics, Inc.” (“Neoleukin”) to “Neurogene Inc.,” and the business conducted by the Company became primarily the business conducted by Neurogene OpCo. Immediately prior to

Closing, the Company effected a 1-for-4 reverse stock split (the “Reverse Stock Split”). Unless noted otherwise, all references in this prospectus to share and per share amounts reflect the Reverse Stock Split.

Concurrently with the execution and delivery of the Merger Agreement, and in order to provide Neurogene OpCo with additional capital for its development programs, Neurogene OpCo entered into a subscription agreement (the “Subscription Agreement”) with certain investors named therein (the “Investors”), pursuant to which, subject to the terms and conditions of the Subscription Agreement, immediately prior to the effective time of the Reverse Merger, Neurogene OpCo issued and sold, and the Investors purchased, 2,792,206 shares of Neurogene OpCo common stock and 1,811,739 pre-funded warrants, exercisable for 1,811,739 shares of Neurogene OpCo common stock, at a purchase price of approximately \$20.63 per share or \$20.63 per warrant, for an aggregate purchase price of approximately \$95.0 million (the “Pre-Closing Financing”).

### **Closing of Private Placement**

On November 5, 2024, we closed a private investment in public equity financing (the “November 2024 private placement”) in which we sold 1,835,000 shares of common stock at a price of \$50.00 per share and, in lieu of shares of common stock, pre-funded warrants to purchase up to an aggregate of 2,165,042 shares of common stock, at a purchase price of \$49.999 per pre-funded warrant, to certain institutional accredited investors. The pre-funded warrants are immediately exercisable until exercised in full at a price of \$0.001 per share of common stock. The aggregate gross proceeds to the Company totaled approximately \$200.0 million. Net proceeds, net of commissions and other offering expenses, are expected to total approximately \$189.5 million.

### **Background**

We were founded in 2018, and have devoted substantially all of our resources to conducting research and development activities (including with respect to the NGN-401 and NGN-101 programs) and undertaking preclinical studies, establishing our manufacturing facility, conducting clinical trials and the manufacturing of product used in our clinical trials and preclinical studies, business planning, developing and maintaining our intellectual property portfolio, hiring personnel, raising capital, and providing general and administrative support for these activities.

Since our inception, we have funded our operations primarily with outside capital (e.g., proceeds from the sale of preferred stock and common stock) and have raised aggregate net proceeds of approximately \$521.9 million from these private placements. However, we have incurred significant recurring losses, including a net loss of \$55.6 million and \$38.7 million for the nine months ended September 30, 2024 and 2023, respectively. In addition, as of September 30, 2024, we had an accumulated deficit of \$242.8 million and cash, cash equivalents and short term investments totaling \$139.0 million. In order to continue our operations, we must achieve profitable operations and/or obtain additional equity or debt financing. Until we achieve profitability, management plans to fund our operations and capital expenditures with cash on hand and the sale and issuance of securities. There can be no assurance that we will be successful in raising additional capital or that such capital, if available, will be on terms that are acceptable to us. If we are unable to raise sufficient additional capital, we may be compelled to consider actions such as reducing the scope of our operations and planned capital expenditures or selling certain assets, including intellectual property assets.

Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on a variety of factors, including the timing, scope and results of our research and development activities. Management expects that our expenses and capital requirements will increase substantially in connection with our ongoing activities as we:

- advance the NGN-401 program through clinical development;
- advance discovery programs from preclinical development into and through clinical development;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- establish sales, marketing and distribution infrastructure to commercialize any approved product candidates;

- establish a commercialization infrastructure and scale up internal and external manufacturing and distribution capabilities to commercialize any product candidates for which we may obtain regulatory approval;
- expand clinical, scientific, management and administrative teams;
- maintain, expand, protect and enforce our intellectual property portfolio, including patents, trade secrets and know-how;
- implement operational, financial and management systems; and
- incur additional legal, accounting and other expenses related to operating as a public company.

We do not have any products approved for commercial sale and have not generated any commercial revenue from product sales. Our ability to generate product revenue sufficient to achieve and maintain profitability will depend upon the successful development and eventual commercialization of one or more of our product candidates, which we expect, if it ever occurs, will take many years. We expect to spend a significant amount in development and marketing costs prior to such time. We will therefore require substantial additional capital to develop our product candidates and support our continuing operations. We may never succeed in achieving regulatory and marketing approval for our product candidates. We may obtain unexpected results from our preclinical and clinical trials. In November 2024, we announced that the Company does not expect to move forward with the NGN-101 CLN5 Batten disease gene therapy program at this time. Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with the FDA. To support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that the application met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. We are currently evaluating options for the program. We may in the future elect to discontinue, delay, or modify additional preclinical and clinical trials of our other product candidates. A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. Accordingly, until such time that we can generate a sufficient amount of revenue from product sales or other sources, if ever, management expects to finance our operations through private or public equity or debt financings, loans or other capital sources, which could include income from collaborations, partnerships or other marketing, distribution, licensing or other strategic arrangements with third parties, or from grants. However, we may be unable to raise additional capital from these sources on favorable terms, or at all. Our failure to obtain sufficient capital on acceptable terms when needed could have a material adverse effect on our business, results of operations or financial condition, including requiring us to delay, reduce or curtail our research, product development or future commercialization efforts. We may also be required to license rights to product candidates at an earlier stage of development or on less favorable terms than we would otherwise choose. Our management cannot provide assurance that we will ever generate positive cash flow from operating activities. See “*Liquidity and Capital Resources.*”

In December 2020, we entered into the Master Research Collaboration (“MCA”) with the University Court of the University of Edinburgh (the “University of Edinburgh”) to support our pipeline development and expansion, and to accelerate scientific innovation to continue to improve upon conventional gene therapy. In November 2023, the collaboration agreement was amended and extended through December 2026. The University of Edinburgh has a vibrant community of over 500 neuroscience researchers and is widely recognized as a preeminent center for neuroscience research, especially in areas of neurodegeneration and in neurodevelopmental disorders, such as Rett syndrome. For example, researchers currently in neuroscience centers at the University of Edinburgh conducted the seminal preclinical work for Rett syndrome, including discovery of the MeCP2 protein, its function as a transcriptional repressor, developing the first and most widely adopted animal model of Rett syndrome, demonstrating for the first time the reversibility of phenotypes in any neurodevelopmental disorder as well as the first ever preclinical gene therapy efforts in Rett syndrome. Under the terms of the agreement, we have the option to in-license product candidates from Dr. Stuart Cobb’s laboratory, where he has a dual appointment as a Professor in Translational Neuroscience at the Patrick Wild Centre and the Centre for Discovery Brain Sciences and serves as our Chief Scientific Officer. Dr. Cobb may be entitled to receive in the future a percentage of certain license-related payments from Neurogene to the University of Edinburgh in accordance with the University of Edinburgh’s standard policies for professor inventors.

## Our Team

Neurogene was founded in January 2018 by Dr. Rachel McMinn with the vision of harnessing the power of gene therapy to turn today's devastating neurological diseases into treatable conditions in the future. We have built a research and development engine through our research collaboration with the University of Edinburgh, which has renowned expertise in neurodevelopmental disorders, and through the leadership of experienced management with extensive expertise in gene therapy manufacturing, we have also developed chemistry manufacturing and controls ("CMC") capabilities and established a fully operational cGMP facility. We are led by a strong management team with deep operational and company building experience as well as significant expertise in research and development in the fields of rare disease and genetic medicine. The members of our management team also provide leadership for certain key functions that are required to develop and obtain regulatory approval for novel treatments, including clinical development and regulatory affairs. Our management team has a deep background of experience in biopharmaceutical companies, including Amicus, AstraZeneca, Auspex, Avexis, Axovant, Cerevel Therapeutics, Eli Lilly, Homology Medicines, ImClone Systems, Intercept Pharmaceuticals, Johnson and Johnson, Lonza, NPS Pharma, Pharmasset, and Takeda. Together, our team has a track record in the discovery, development, and commercialization of multiple therapies for devastating disorders.

## Our Approach

We have a bold vision to harness the power of gene therapy together with our EXACT technology to turn today's complex devastating neurological diseases into treatable conditions. Fundamental to accomplishing this goal are three capabilities that provide us with important competitive advantages that we believe support our disciplined product development approach and improve the probability of technical and regulatory success of our product candidates.

- 1. *Our EXACT Technology.*** We developed the EXACT technology, in collaboration with the University of Edinburgh, with the goal of solving the problem of variable gene expression resulting from the inherent limitations we believe exist with conventional gene therapy. We believe our EXACT technology has the potential to overcome this challenge by widening the otherwise narrow therapeutic window for transgene expression in certain complex neurological diseases. The EXACT technology is predicted to be delivery agnostic and compatible with viral and non-viral delivery platforms.
- 2. *Optimal Drug Delivery Approaches to Treat CNS Disorders.*** We believe in utilizing the most optimal routes of administration to deliver our product candidates which we believe will best target the underlying pathophysiology and biology of the disease. We rigorously study potential central nervous system ("CNS") indications and their underlying pathologies prior to choosing a candidate and deliberately choose what we believe is the most appropriate route of administration to increase the probability of technical and regulatory success.
- 3. *Scalable and Flexible Manufacturing.*** We believe that integrating in-house cGMP manufacturing capabilities enables superior oversight of product quality and greater control of development timelines, allows for strategic pipeline flexibility, and promotes continuity in our process from preclinical to clinical to commercial manufacturing in the future. Besides cGMP manufacturing, our core development capabilities include quality control, process, analytical, and bioanalytical development labs with experienced teams. We believe that our in-house manufacturing capabilities also possess the potential to avoid comparability challenges caused by the introduction of significant platform-based changes during the product development phase that other gene therapy companies have encountered. We believe our in-house manufacturing also provides increased flexibility to manufacture products more efficiently and more cost effectively.



## Neurogene's EXACT Technology Acts as a Genetic Thermostat



EXACT's transgene control elements consist of an embedded non-mammalian miRNA, and its complementary recognition sites. This combination is designed to avoid off-target gene regulation. The transgene and the miRNA are co-expressed from the same construct under the control of the same promoter. Because the miRNA and recognition sites are fully complementary with no mismatches, the miRNA-bound transcripts are predicted to be rapidly destroyed, limiting the number of available transgene mRNA copies. These remaining mRNA transcripts are then translated into transgene derived protein. Importantly, the more transgene that is expressed in a given cell, the more miRNA that is produced simultaneously, leading to greater destruction of transcripts. This relationship ultimately creates a genetic thermostat, which attenuates transgene expression, and thereby is designed to avoid the significant toxicity associated with variable gene expression related to conventional gene therapy.

The non-mammalian miRNA regulatory element that is part of an EXACT gene circuit is designed to minimize affinity to human mRNAs, and thereby avoid off-target gene regulation. We believe such construct design elements differentiate our EXACT technology from other miRNA mediated regulation approaches. The EXACT technology is also predicted to be delivery agnostic and compatible with viral and non-viral delivery platforms.

Using EXACT, we are advancing a pipeline of therapeutic programs intended to treat complex neurological disorders that we believe will not be suitable for treatment with conventional gene therapy.

### Our Pipeline

#### NGN-401

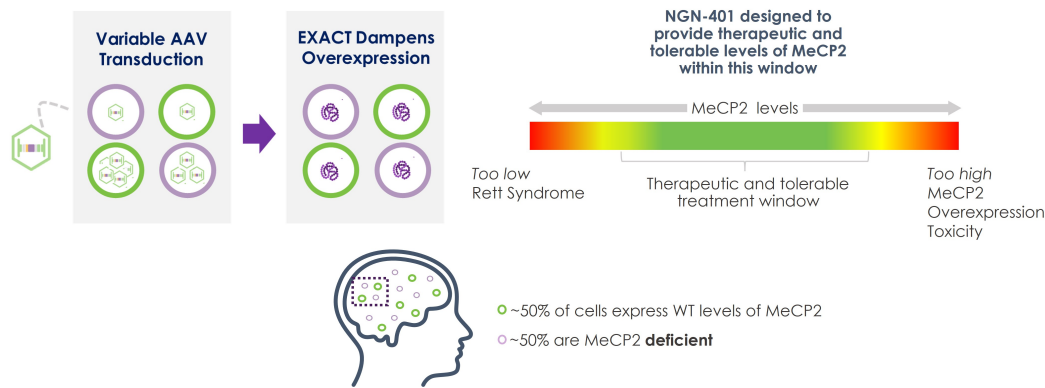
Our first clinical-stage program to utilize the EXACT platform is NGN-401, which is in development for the treatment of Rett syndrome, a disease with a patient population that has a significant unmet need, and that ultimately progresses to substantial neurological and physical impairment and premature death. NGN-401 is packaged in an adeno-associated virus 9 ("AAV9") capsid. In preclinical studies, we assessed a single ICV administration of NGN-401 in multiple preclinical models, including the male knockout mouse model for efficacy, the female *Mecp2* mouse model for tolerability, and non-human primates ("NHPs") for biodistribution and toxicity.

Rett syndrome is an X-linked neurodevelopmental disorder caused by a pathogenic mutation in one copy of the *MECP2* gene that leads to deficiency of the MeCP2 protein in approximately 50% of cells. MeCP2 is a critical protein responsible for normal function in the brain and other parts of the nervous system. Rett syndrome has an estimated global incidence of 1 in 10,000 females. In the United States, the prevalence of Rett syndrome is estimated to be approximately 6,000 to 9,000 patients. The estimated prevalence in the European Union ("EU") and select foreign countries is estimated in total to be greater than in the United States. Rett syndrome in females is marked by several cardinal clinical features, including significant impairments in communication (for example, an inability to communicate verbally or with their hands), gross and fine motor function, autonomic function, and a range of other

disease manifestations. While there is one treatment approved to treat Rett syndrome, there remains a significant unmet need for new treatment options that target the root cause of the disease.

Rett syndrome as modeled in mice has been shown to be inducible and reversible, demonstrating that the MECP2 gene is critical throughout lifespan and offering the prospect of disease reversibility in humans. However, gene replacement therapy is not straightforward for Rett syndrome because too little MeCP2 causes Rett syndrome, while too much MeCP2 causes a similarly devastating disease known as MECP2 duplication syndrome. This MECP2 gene sensitivity results in a narrow therapeutic window for gene therapy in Rett syndrome. Therefore, we believe the goal in developing a gene replacement therapy for Rett syndrome is to supply enough MeCP2 to deficient cells, without causing toxicity to healthy cells. Achieving this goal requires precise control over the level of MECP2 expression on a cell-by-cell basis. We designed EXACT with achieving this goal in mind, and have selected Rett syndrome as the indication for our first EXACT product candidate.

### **EXACT Technology for Rett Syndrome**



As shown in the left-hand panel above, an inherent limitation of AAV administration is that it produces variable levels of transduction across cells. Despite this variability, our preclinical data demonstrate the potential of EXACT to normalize the levels of MeCP2 protein (middle panel). The right-hand panel illustrates the aspiration of EXACT for Rett syndrome—to deliver transgene levels of functional MeCP2 that, when expressed on top of endogenous levels, can be well tolerated, while simultaneously delivering a therapeutically relevant level of MeCP2 to deficient cells to allow for efficacy. We believe our EXACT technology has the potential to overcome the limitations of the narrow therapeutic window for gene therapy in Rett syndrome and offers the possibility of making gene replacement a viable modality to treat complex disorders such as Rett syndrome.

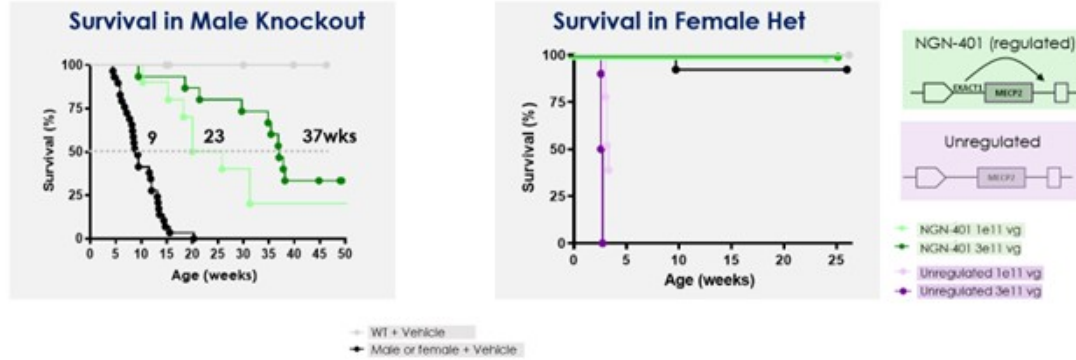
### **NGN-401 Product Design**



NGN-401 contains the EXACT regulatory elements shown above, which regulate the expression of the full-length human MECP2 gene. Expression is driven by a mammalian promoter that has been used in gene therapy clinical trials. This genetic construct is packaged into an AAV9 capsid.

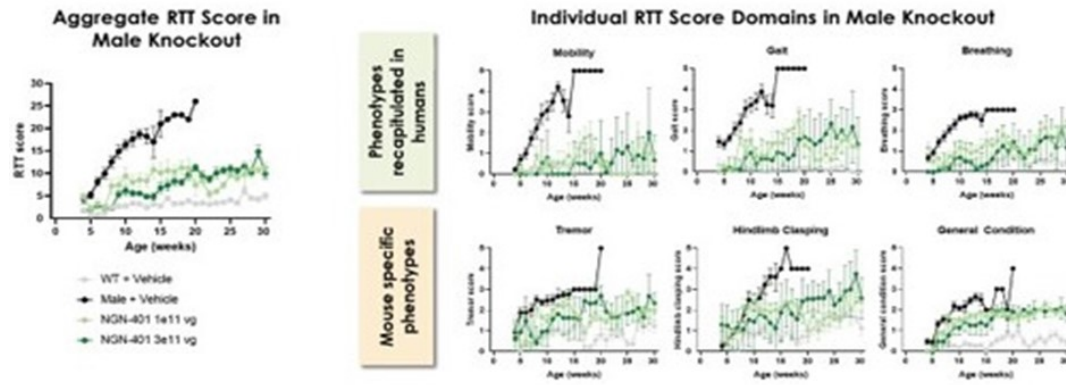
**NGN-401 Preclinical Data in Rett Syndrome Mouse Models**

The male knockout mouse is the gold standard for evaluating efficacy in Rett syndrome because it has a robust phenotype that expresses certain cardinal features of Rett syndrome, including motor, gait, and breathing abnormalities. This model has a more severe disease course compared to human females with Rett syndrome, because 100% of male mouse cells are MeCP2 deficient. In contrast, human females have MeCP2 deficiency in approximately 50% of cells, due to a mosaic pattern of X-inactivation whereby the healthy MECP2 gene or the pathogenic gene is randomly selected to be silenced. Signs of disease in the male mouse model have been documented in the early post-natal period, with mice beginning to die or reach the humane endpoint as early as four to five weeks of life, with a median survival of approximately nine weeks.



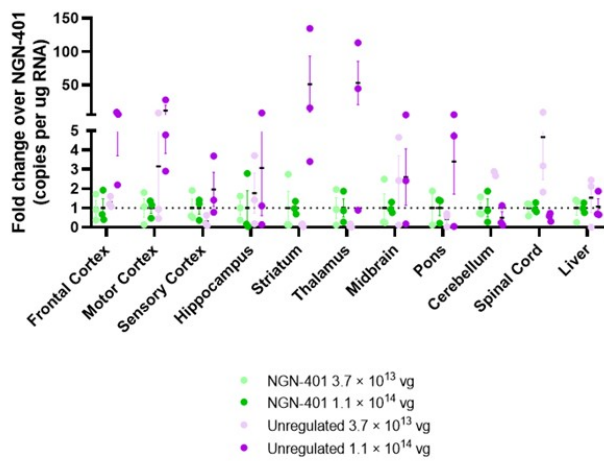
In the preclinical study described above, male knockout mice were administered a one-time administration of NGN-401 in the early postnatal period using an ICV procedure with either vehicle, or product doses of 1e11 or 3e11 vector genomes (“vg”) per mouse. The profile for NGN-401 in this mouse model demonstrated a dose-dependent improvement in survival (shown in the left-hand panel above) with concomitant improvements in Rett syndrome-like phenotypes compared to vehicle treated control animals.

To test tolerability, we evaluated the same doses of NGN-401 that demonstrated dose-dependent improvement in the male mouse model in a female mouse model (shown in the right-hand panel above). These female mice are genotypically comparable to human female patients, allowing us to demonstrate tolerability where approximately 50% of cells have normal levels of MeCP2 expression. NGN-401 (shown in green above) was well tolerated, with no negative effects on survival. In comparison, when we conducted a similar experiment using conventional gene therapy (depicted in purple above), which we refer to as “unregulated,” these mice experienced rapid toxicity and died or reached the humane endpoint within two to three weeks. These deaths were associated with significant overexpression of MeCP2, demonstrating the importance of controlling MECP2 transgene expression to provide tolerable protein levels, which we believe the EXACT technology is able to accomplish.

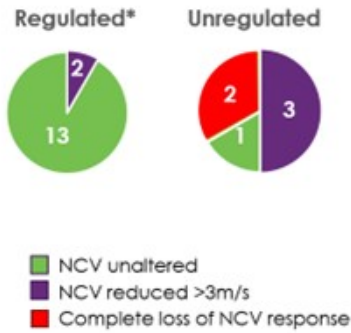


In the male knockout mouse model, phenotypic improvement was measured using an observational scoring system evaluating six disease phenotypes (referred to as the “RTT Score”). The aggregate observational score, shown in the left panel above, was improved with NGN-401 treatment, with the greatest amelioration of symptoms observed in translationally relevant domains of mobility, gait, and breathing (shown in the top right panel above).

***NGN-401 Preclinical Wild-Type Non-Human Primate 30-day Data***



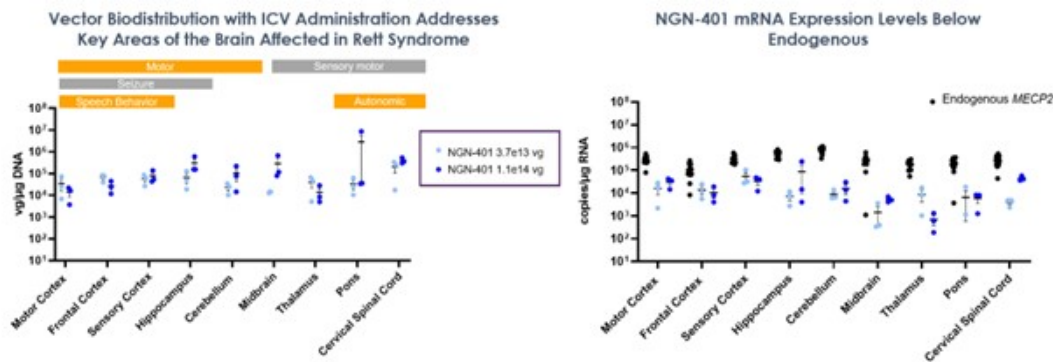
In an NHP study shown in the figure above, we evaluated NGN-401 and unregulated conventional gene therapy constructs that do not contain EXACT regulatory elements. The first NHP study was a 30-day expression and tolerability study, comparing the same doses of NGN-401 to the unregulated control. Results from this study show tight mRNA expression levels of NGN-401, with greater and more variable expression for the unregulated vector.



- Regulated includes NGN-401 and another EXACT vector; data at 30 days

NCV: nerve conduction velocity

These higher levels of MeCP2 in the unregulated treatment group were associated with early signs of toxicity in NHPs, shown by reduced or complete loss of sural nerve function as measured by nerve conduction velocity in most animals.



We also evaluated vector biodistribution and MECP2 mRNA for NGN-401 treated animals to assess how biodistribution and expression maps to key brain regions underlying Rett syndrome pathobiology in NHPs. We found that NGN-401 viral biodistribution tracks to key areas of the brain and spinal cord that underlie cardinal features of Rett syndrome, including speech, motor and autonomic function (shown in the left-hand panel above). In addition, we observed significant levels of transgene-derived MECP2 mRNA in key brain regions (shown in the right-hand panel above), supporting the prospect for therapeutic relevance to cardinal features of human disease and is consistent with the phenotypic improvements observed in the male knockout mouse model. We also observed that MECP2 mRNA levels derived from NGN-401 were below endogenous wild type NHP MECP2 levels, underscoring the potential safety profile of dampened MECP2 expression in these animals.

In a second NHP study, we conducted a Good Laboratory Practice (“GLP”) toxicology study with cohorts at two doses with endpoints at three and six months. This study assessed but found no signs or symptoms of MECP2

overexpression with a >4x safety margin from our starting clinical dose. Instead, we observed a typical profile for AAV9 administered product, with minimal to slight microscopic findings in the dorsal root ganglion (“DRG”), spinal cord, brain, and peripheral nerves. These observations were not dose dependent and there were no related clinical observations. In the >4x dose, one out of six NHPs had a loss of sural nerve conduction as measured by nerve conduction study. Early transient aminotransferase elevations were also observed, which returned to baseline or near baseline values within approximately the first three weeks following NGN-401 administration and prednisolone prophylaxis, without the need for additional immunosuppression. Based on these findings, the high dose cohort was considered the no-observed adverse effect level (“NOAEL”). Both of these observations (liver and DRG) are commonly observed in NHP models with AAV administration. While liver enzyme elevations have been connected with liver toxicity for certain gene therapy products in humans, these findings are typically resolved in humans without clinical sequelae.

#### ***NGN-401 Phase 1/2 Clinical Trial***

We are conducting a Phase 1/2 open-label, multi-center clinical trial of NGN-401 gene therapy for the treatment of female patients with classic Rett syndrome that is assessing the safety, tolerability, and efficacy of NGN-401 at a dose of 1E15 vg. In November 2024, we announced the initiation of an adolescent/adult cohort that will evaluate NGN-401 in participants ages 16 and above. Clinical grade NGN-401 was manufactured at our manufacturing facility and is being used for dosing in the Phase 1/2 clinical trial. NGN-401 is delivered using a one-time intracerebroventricular (“ICV”) procedure, which we believe is the most suitable route of administration to achieve optimal biodistribution in key regions of the brain and other parts of the nervous system.

The NGN-401 clinical trial has been initiated in three regions - the United States, the United Kingdom (“UK”) and Australia, after clearance of our Investigational New Drug (“IND”) application by the U.S. Food and Drug Administration (“FDA”) in January 2023, the clearance of the clinical trial application by the UK Medicines and Healthcare Products Regulatory Agency in January 2024, and acknowledgement from the Australian Therapeutic Goods Administration and approval from the Human Research Ethics Committee in May 2024. Participants have been dosed in all three regions.

On November 11, 2024, we announced positive interim clinical data in all participants in the 1E15 vg cohort (low-dose cohort, n=4) from the Phase 1/2 clinical trial with a data cut-off date of October 17, 2024. Consistent, concordant and durable improvements were observed as measured from baseline across multiple Rett syndrome clinical assessments. Participants also achieved meaningful gains of skills and developmental milestones in hand function, gross motor, and communication, all of which are core clinical domains of Rett syndrome. These gains of skills and developmental milestones are not expected to occur when compared and contextualized against the natural history of Rett syndrome. Additionally, objective improvements in autonomic function were recorded.

Consistent improvements were observed in the following:

	CGI-I		CGI-S Total Score		RSBQ		Gain of Skills, Developmental Milestones and Symptom Improvement in RIT Clinical Domains				
	Improved?	How many points?*	Improved?	How many points?	Improved?	How many points? (% Change)	Hand Function	Gross Motor	Communication	Autonomic	Attentiveness
LD:1 15 mos. post-NGN-401	✓	2 pts.			✓	10 pts. (-28%)	✓	✓	✓	✓	✓
LD:2 12 mos. post-NGN-401	✓	2 pts.	✓	1 pt.	✓	32 pts. (-52%)	✓	✓	✓	✓	✓
LD:3 9 mos. post-NGN-401	✓	2 pts.			✓	5 pts. (-29%)	✓	✓		✓	✓
LD:4 3 mos. post-NGN-401	✓	2 pts.			✓	8 pts. (-28%)	✓			✓	✓

As of data cut-off date of October 17, 2024

\*Each participant achieved a 2-point improvement or “much improved” from baseline.

CGI-I = Clinician Global Impression-Improvement; CGI-S = Clinician Global Impression-Severity; RSBQ = Rett Syndrome Behavior Questionnaire

We also announced safety and tolerability data from the Phase 1/2 clinical trial with a data cut-off date of October 17, 2024. NGN-401 has been well-tolerated and shows a favorable safety profile at a dose of 1E15 vg (n=5, low-dose cohort), with no treatment-related serious adverse events (“SAEs”) at that dose. Treatment-related AEs at the 1E15 vg dose have been mild/Grade 1. One Grade 1 AE of abnormal sural (sensory) nerve conduction study at the 1E15 vg dose level was observed, and the participant is asymptomatic. Treatment-related AEs in the first two participants dosed with 3E15 vg of NGN-401 (high-dose cohort) included Grade 2 and Grade 3 elevations in alanine aminotransferase and aspartate aminotransferase levels, and Grade 2 decreased platelets, which resolved with steroids. One Grade 1 AE of abnormal sural (sensory) nerve conduction study at the 3E15 vg dose level was observed, and the participant is asymptomatic. Across both dose levels, there were no signs or symptoms indicating MeCP2 overexpression toxicity, and there were no ICV procedure-related AEs.

On November 11, 2024, we were notified of an SAE in the third participant who was dosed with 3E15 vg (high-dose cohort) on November 5, 2024. The participant subsequently died following complications from a rare and life-threatening hyperinflammatory syndrome associated with systemic exposure to high doses of AAV. Upon notification of the SAE, the Company consulted with the Data Safety Monitoring Board (DSMB) regarding the continued dosing of participants in the 1E15 vg (low-dose cohort). Following this consultation, the DSMB did not express concerns regarding the continued dosing of participants in the low-dose cohort. The Company also paused further use of the 3E15 vg dose (high dose cohort) on November 11, 2024 and does not plan to enroll any further participants at that dose level. The U.S. Food and Drug Administration (“FDA”) completed a review of the safety data for NGN-401 and allowed us to proceed with the Phase 1/2 trial using the 1E15 vg dose for both the pediatric and adolescent/adult cohorts. We are updating the trial protocol to remove the 3E15 vg dose. We will also incorporate the 1E15 vg dose in our future registrational trial design planning

The baseline demographics of the first five participants who received NGN-401 in the 1E15 vg (low-dose) Cohort 1, and the first two participants who received NGN-401 in the 3E15 vg (high dose) Cohort 2 include:

	Low-Dose Cohort 1 (1E15 vg)					High-Dose Cohort 2 (3E15 vg)	
	Participant 1 (LD:1)	Participant 2 (LD:2)	Participant 3 (LD:3)	Participant 4 (LD:4)	Participant 5 (LD:5)	Participant 1 (HD:1)	Participant 2 (HD:2)
Age at Dosing in Years	7	4	6	7	6	5	7
MECP2 Mutation Severity	Mild	Severe	Severe	Severe	Severe	Severe	Unclassified
Baseline Disease Severity as Indicated by CGI-S Score	4 (moderately ill)	5 (markedly ill)	5 (markedly ill)	5 (markedly ill)	5 (markedly ill)	5 (markedly ill)	4 (moderately ill)
Time Post Treatment with NGN-401 in Months	~15	~12	~9	<6	~1	~5	~2

In November 2024, we also shared that we have gained alignment with the FDA on our potency assay strategy and CMC scale-up planning for the program. We expect to share information about our registration trial design in the first half of 2025 and to announce additional Phase 1/2 clinical data in the second half of 2025.

In June 2024, we also announced that NGN-401 was one of four sponsors selected by the Center for Biologics Evaluation and Research at the FDA to participate in the FDA’s Support for clinical Trials Advancing Rare disease Therapeutics (“START”) Pilot Program based on potential for clinical benefits and clinical development program readiness. As part of the START Program, we have opportunities for enhanced communications with the FDA, with the aim to further accelerate the pace of NGN-401’s development. These opportunities are designed to provide frequent advice and regular ad-hoc conversations to address product-specific development issues, including, but not limited to, clinical study design, choice of control group and fine-tuning the choice of patient population. In August 2024, we announced that NGN-401 also received Regenerative Medicine Advanced Therapy (“RMAT”) designation from the FDA. RMAT designation is granted for regenerative medicines intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition, and with preliminary clinical evidence that indicates that the drug has the potential to address unmet medical needs. Benefits of the RMAT designation program include all the benefits of Fast Track and Breakthrough Therapy designation programs, including early and frequent communications with FDA senior managers, intensive guidance on efficient drug development and eligibility for an Accelerated Approval pathway and Priority Review.

### ***EXACT Discovery Pipeline***

We have a skilled team of scientists, both internally and in conjunction with our collaboration with the University of Edinburgh, with extensive gene therapy experience. Our team is focused on expanding our transgene regulation pipeline and leveraging EXACT beyond its NGN-401 clinical candidate for Rett syndrome. We believe that our EXACT platform has broad applicability in complex neurological diseases not otherwise easily addressable by conventional gene therapy. In addition to our Rett syndrome program, we have multiple programs in the discovery stage. We anticipate advancing one program into clinical development in 2025.

### ***NGN-101***

NGN-101 is our non-EXACT, conventional gene therapy development candidate for the treatment of CLN5 Batten disease, designed to deliver the CLN5 gene and packaged in an AAV9 capsid. We have completed enrollment in a Phase 1/2 clinical trial for NGN-101 using a dual route of administration to deliver NGN-101 via single ICV and intravitreal (“IVT”) administrations to treat the neurodegenerative and ocular disease manifestations of Batten in pediatric patients between the age of three and nine years old who have a confirmed genetic diagnosis of CLN5 Batten disease. In November 2024, we announced that the Company does not expect to move forward with the NGN-101 CLN5 Batten disease gene therapy program at this time. Given the rarity of the disease, continued investment in the program was predicated on alignment on a streamlined registrational pathway with the FDA. To



support a streamlined pathway, we submitted an RMAT application to the FDA. Despite our belief that we met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied. We are currently evaluating options for the program.



In preclinical studies, we assessed a single ICV administration, a single IVT administration, and the combination of ICV and IVT dosing of NGN-101 in a CLN5 Batten sheep model for efficacy and NHPs for biodistribution and toxicity.

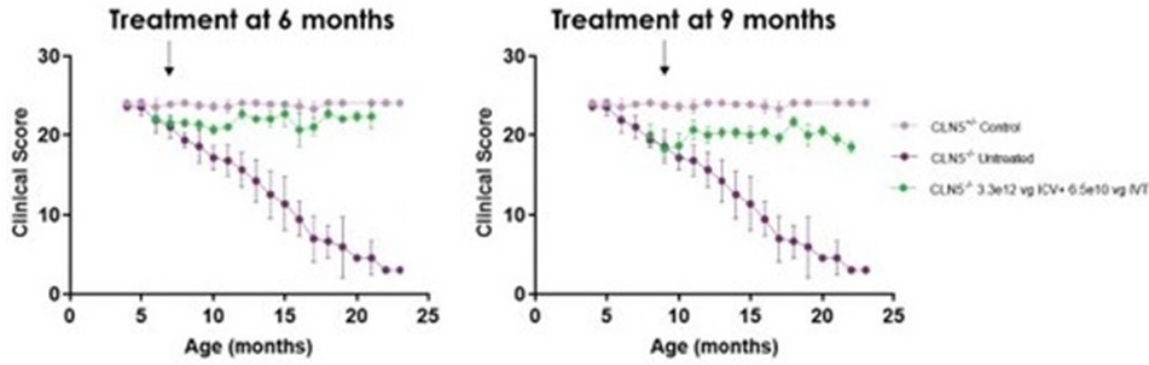
Batten disease is a family of rare neurodegenerative diseases caused by pathogenic changes in one of a series of genes that results in the accumulation of toxic deposits across multiple organ systems. CLN5 Batten disease is a rare, pediatric-onset and rapidly progressive condition caused by a pathogenic mutation in the CLN5 gene, leading to loss of function. It is characterized by loss of vision, seizures, and progressive decline in intellectual and motor capabilities beginning in childhood, leading to substantial impairments and early mortality. The incidence of Batten disease is estimated to be 1:100,000, with CLN5 Batten disease a small subset.

Currently, CLN5 Batten disease has no approved disease-specific treatment options.

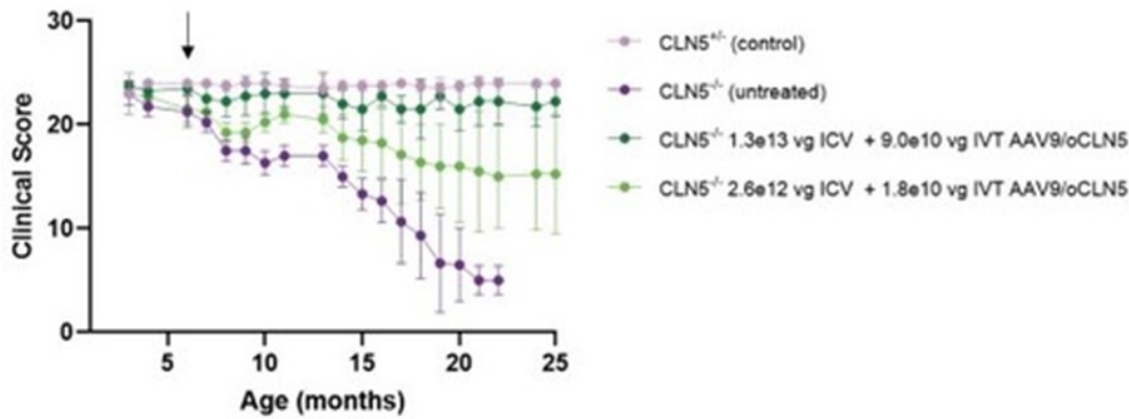
### ***Preclinical CLN5 Data in Sheep Disease Model***

There is a naturally occurring Borderdale sheep model that is deficient in the CLN5 protein and shows several of the symptoms found in human CLN5 Batten disease, including progressive vision loss, motor and gait abnormalities, and premature death. These animals typically reach the humane endpoint by 16-19 months, with a maximal life expectancy of 22 months. A clinical scoring system was developed by Lincoln University comprising 10 physical domains to evaluate the disease phenotype of these sheep, although only six domains developed a progressive change over time in the disease model. Data for a modified ovine Batten disease rating scale, consisting of the cumulative score of these six domains, are shown below, with a score of 24 reflecting a phenotypically normal animal.

In our preclinical study, sheep were administered a one-time IVT administration alone, ICV administration alone, or combination of IVT and ICV administration of an AAV9 vector containing an ovine version of the CLN5 transgene (AAV9/oCLN5). AAV9/oCLN5 slowed or halted key features of disease progression in the naturally occurring CLN5-deficient sheep model. While IVT administration alone preserved retinal layers within the eye, sheep ultimately succumbed to neurological disease and did not experience a survival benefit. In comparison, ICV administration alone significantly extended survival, but sheep experienced vision loss and blindness and subsequently experienced loss of function in other domains. Animals treated with concurrent administration employing both ICV and IVT routes of delivery experienced the most robust survival and phenotypic benefits, including preservation of translationally relevant phenotypes—visual and motor function.

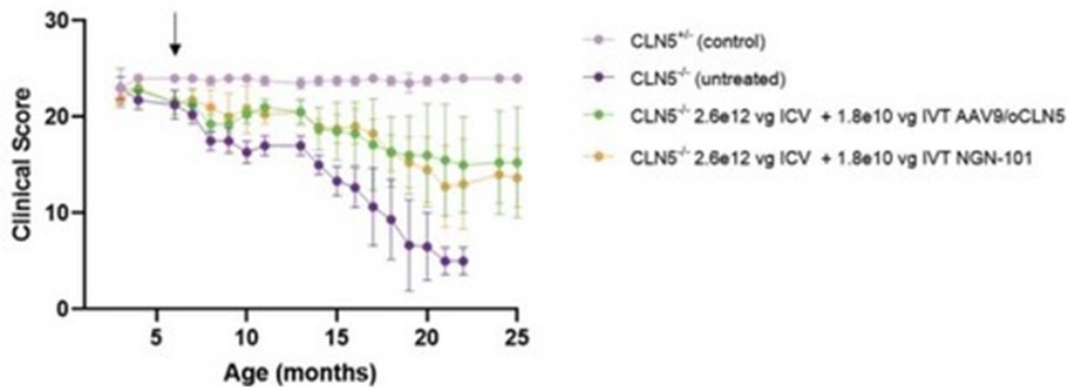


Animals that received AAV9/oCLN5 at either an early symptomatic (six months of age) or advanced symptomatic (nine months of age) disease state exhibited delayed disease progression and stabilized clinical function, demonstrated above in the clinical scoring data. AAV9/oCLN5 treatment preserved vision in the treated eye and mitigated declines in body weight, intracranial volume, and retinal function, as well as ameliorated brain and retinal pathology.



We conducted an additional study (shown above) in the ovine model of CLN5 disease to evaluate an approximately 4x higher ICV dose of AAV9/oCLN5 administered at an early symptomatic disease stage (six months of age), as well as to further explore IVT dosing. Data in this preclinical model demonstrated that the 4x higher ICV dose of AAV9/oCLN5 along with a modest IVT dose increase was generally well tolerated in this disease model and demonstrated an improved therapeutic effect as compared to the cohort of ovine animals receiving a similar ICV dose as previously tested, and approximately only 30% of the IVT dose. These data provided additional support for our clinical strategy for dose escalation in our ongoing Phase 1/2 clinical trial of NGN-101.

*Bridging Sheep Study Comparing Ovine and Human CLN5 Transgene Administration*

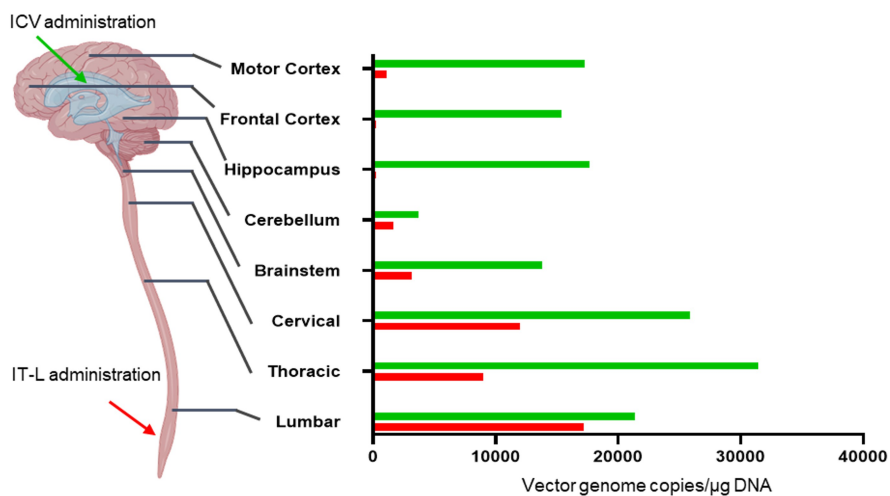


We also conducted a bridging sheep efficacy study that compared equivalent doses of AAV9/oCLN5 to NGN-101, the clinical product candidate containing the human CLN5 transgene. Based on the results of this study, we found the phenotypical improvements observed in the CLN5 knockout sheep were similar between the two transgenes.

We conducted a GLP toxicology and biodistribution study with NGN-101 in NHPs with three- and six-month timepoints. NGN-101 was generally well-tolerated in this study. Non-adverse transient aminotransferase elevations were observed, which were not associated with anatomic pathology findings. In addition, one animal inadvertently dosed with a two-fold higher dose than our highest planned IVT clinical dose experienced a moderately severe intraocular inflammatory response, which subsided with anti-inflammatory treatment. Based on these findings, the high dose cohort was considered the NOAEL.

**Utilizing Optimal Drug Delivery to Treat CNS Disorders**

To increase the probability of technical and regulatory success, we believe in utilizing the most optimal route of administration for AAV9 that best targets the underlying pathophysiology and biology of the disease.



The pathobiology of both Rett syndrome and CLN5 Batten disease involves structures across the nervous system. Therefore, it was critical to us to evaluate the optimal route of administration to achieve broad AAV9 distribution to key regions relevant for disease. To better appreciate each route of administration and to take a

rational approach to choosing the optimal route of administration for our programs, we conducted a one-month study to evaluate a single total dose of an AAV9 vector containing a human CLN5 transgene comparing multiple routes of administration in NHPs. Shown above are vector genome biodistribution data specifically for unilateral ICV and intrathecal lumbar (“IT-L”) delivery. ICV administration, shown in green, demonstrated broad biodistribution throughout the brain and spinal cord. When compared to IT-L delivery (shown in red above), ICV delivery achieved distribution that was significantly better to key areas of the nervous system underlying Rett syndrome and CLN5 Batten disease pathology. These areas include the cortex, hippocampus, and areas of the brain stem (pons and medulla). These data supported the selection of the ICV route of administration for the NGN-401 and NGN-101 programs.

### **Intellectual Property**

We actively seek to protect our proprietary technology, inventions, and other intellectual property that is commercially important to the development of our business by a variety of means, including seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. In particular, our patent strategy includes the filing of patent applications covering regulatory elements embodied by our EXACT technology and our unique gene sequences. We also may rely on trade secrets and know-how relating to our proprietary technology platform, including our EXACT platform technology, on continuing technological innovation and on in-licensing opportunities that may be important for the development of our business to develop, strengthen and maintain the strength of our position in the field of gene therapy. We also intend to seek patent protection or rely upon trade secret rights to protect other technologies that we may use to discover and validate targets, and that we may use to manufacture and develop novel gene therapy products. We are a party to license agreements that give us rights to use specific technologies in our gene therapy products and in manufacturing our products. Additional regulatory protection may also be afforded through data exclusivity, market exclusivity and patent term extensions where available.

As of September 30, 2024, Neurogene licenses 29 patent applications, including Patent Cooperation Treaty (“PCT”), U.S., and international patent applications as described below. Our policy is to file patent applications to protect technology, inventions and improvements to inventions that may be commercially important to the development of our business. Patent applications and patents directed to specific product candidates are summarized below:

#### ***EXACT Technology***

We in-license from the University of Edinburgh 12 pending patent applications worldwide directed to regulatory control of transgene expression (including composition of matter, use, and process of making the therapeutic products). Any patents based on these applications, if issued, are expected to expire in 2041, without taking into account any possible patent term adjustment, regulatory extensions, or terminal disclaimers, and assuming payment of all annuities and/or maintenance fees.

#### ***NGN-401 for Rett Syndrome***

We also in-license from the University of Edinburgh twelve pending patent applications worldwide directed to recombinant MECP2 therapeutic constructs and methods for treating Rett syndrome and related conditions (including composition of matter, use, and process of making the therapeutic products). Any patents based on this application, if issued, are expected to expire in 2043, without taking into account any possible patent term adjustment, regulatory extensions, or terminal disclaimers, and assuming payment of all annuities and/or maintenance fees.

#### ***NGN-101 for CLN5 Batten Disease***

We in-license from the University of North Carolina at Chapel Hill five pending patent applications worldwide directed to an optimized CLN5 therapeutic construct for treating Batten disease (including composition of matter, use, and process of making the therapeutic products). Any patents based on these applications, if issued, are expected to expire in 2039, without taking into account any possible patent term adjustment, regulatory extensions, or terminal disclaimers, and assuming payment of all annuities and/or maintenance fees.

Individual patents extend for varying periods depending on the date of filing of the patent application or the date of patent issuance and the legal term of patents in the countries in which they are obtained. Generally, patents issued for regularly-filed applications in the U.S. are effective for 20 years from the earliest effective non-provisional filing date. In addition, in certain instances, a patent term can be extended to recapture a portion of the U.S. Patent and Trademark Office's delay in issuing the patent as well as a portion of the term effectively lost as a result of the FDA regulatory review period. However, as to the FDA component, the restoration period cannot be longer than five years and the total patent term including the restoration period must not exceed 14 years following FDA approval. The duration of foreign patents varies in accordance with provisions of applicable local law, but typically is also 20 years from the earliest effective filing date. The actual protection afforded by a patent varies on a product-by-product basis, from country to country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent.

We also protect our trade secrets and other proprietary technology and processes, in part, by confidentiality and invention assignment agreements with our employees, consultants, scientific advisors and other contractors. These agreements may be breached, and we may not have adequate remedies for breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our employees, consultants, scientific advisors, or other contractors use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Our commercial success will also depend in part on not infringing the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent would require us to alter our development or commercial strategies, alter our drugs or processes, obtain licenses, or cease certain activities. Our breach of any license agreements or failure to obtain a license to proprietary rights that we may require to develop or commercialize our future drugs may have a material adverse impact on our business, operations and financial condition.

## **Employees and Human Capital Resources**

### ***Our Values***

Neurogene was founded on a passionate belief that innovation in gene therapy can bring treatment options to patients with complex neurological diseases—patients who are waiting with unmet needs and often overlooked. Our behaviors reflect our values and encompass how our teams work together with open minds, reimagining the future and advocating for patients and families to achieve our mission. Our vision is to turn devastating neurological diseases into treatable conditions and improve the lives of patients and their families. We are focused on building a corporate culture that nurtures innovation, creative problem solving and a strong sense of purpose with patient and caregiver mindsets at the forefront. Our core values include:

- **Patients and Families are Waiting:** We do what is right for our patients, our teams and our community
- **It's Better Together:** We are passionate about our work, our colleagues and our patients, caregivers and families
- **Keep an Open Mind:** We actively listen to and value diverse opinions
- **Reimagine the Future:** We drive, innovate, take balanced risks and advance therapies with a sense of urgency

We seek to prioritize employee development and align employees' goals with our vision, mission, and overall strategic direction. Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards and cash-based performance bonus awards, in order to align such individuals' goals with increasing stockholder value and the success of Neurogene.

As of November 15, 2024, we had 100 employees, all of whom were employed full time and 76 of whom were engaged in research, development and technical operations activities. Twenty-two of our employees hold Ph.D. or M.D. degrees. None of our employees are represented by a labor union or covered under a collective bargaining agreement. We consider our relationship with our employees to be good.

### **Commercial**

Should any of our product candidates be approved for commercialization, we intend to develop a plan to commercialize them in the U.S. and other key markets, through internal infrastructure and/or external partnerships in a manner that will enable us to realize the full commercial value of our programs. Given the company's stage of development, we have not yet established a commercial organization or distribution capabilities. We currently hold worldwide development and commercialization rights, including through exclusive licenses, to all of our product candidates.

### **Manufacturing**

Our fully-operational, cGMP manufacturing facility is located in Houston, Texas, and includes process and analytical development labs. The site is approximately 42,000 square feet, with 6,000 square feet of cleanroom space dedicated to cGMP production of clinical product. Our manufacturing facility is also designed for commercial-grade drug product in the future (if regulatory approval is obtained). The facility includes our experienced team of approximately 55 employees that support process development, analytical development, quality assurance, quality control, manufacturing, supply chain, and maintenance. In addition to our process and analytical development capabilities, we have established a bioanalytical group that allows us to analyze vector biodistribution, mRNA expression, and protein expression from in-vivo preclinical studies. We believe this internal capability provides us with a lower cost structure and greater control over timelines driven by execution of our corporate priorities through dedicated oversight by our employees. We have produced nonclinical material to support our preclinical studies, including product candidates manufactured for use for IND-enabling studies.

We believe that our in-house manufacturing capabilities enable us to control product quality and development timelines, allow for strategic pipeline flexibility, and provide us with continuity in our process from preclinical to clinical to commercial manufacturing in the future (if regulatory approval is obtained). With in-house manufacturing capabilities designed to transition from preclinical to clinical-stage trials, beginning with NGN-401 for the treatment of Rett syndrome, we believe we are well positioned to avoid future product comparability challenges that other gene therapy companies have faced. NGN-401 has been successfully manufactured at our manufacturing facility and clinical-grade product is available for dosing in our ongoing Phase 1/2 clinical trial for females with Rett syndrome that is currently enrolling patients. We expect to manufacture in our facility subsequent cGMP campaigns for NGN-401, in addition to those for our early discovery pipeline. We believe internalizing our manufacturing capabilities has two significant financial advantages: (1) it provides us with the potential to have maximum flexibility to manufacture product candidates at a reduced cost and (2) it affords us greater control over CMC investments as programs progress through development.

The manufacturing facility is designed to be flexible, scalable, and a multi-product facility that can support two major scalable AAV production processes: transient transfection process using mammalian cells (HEK293) and an insect cell (Sf9) baculovirus based AAV production system. The processing suites are fitted with equipment that supports single-use technology, which we believe reduces the risk of cross-contamination and allows for multiple products to be manufactured utilizing either process. In addition, we designed the fill-finish suite to allow for final product to be vialled in-house, which we believe eliminates the need for a contract manufacturer and excessive shipping of product between facilities. AAV9 vector intended for IND-enabling studies is generated utilizing the same platform process (either HEK293 or Sf9 based) that is expected to be used in the clinic up to a 50L scale in our process development labs. Based on current program needs, the cGMP platform processes are executed at a 200L scale, and we believe we have the ability to scale up or scale out as material needs increase.

### **Competition**

The biotechnology and pharmaceutical industries generally, and the gene therapy field specifically, are characterized by rapid evolution of technologies, competition and strong defense of intellectual property. Any

product candidates that we develop and commercialize will face competition from existing therapies and new therapies that may become available in the future. While we believe our products, technology, scientific knowledge, talent and manufacturing capabilities differentiate us and provide us with competitive advantages, we face competition from other biotechnology companies, pharmaceutical and specialty pharmaceutical companies, as well as academic institutions. Our ability to compete will significantly depend upon our ability to complete necessary clinical trials and regulatory approval processes, and effectively market any drug that we may successfully develop.

While no disease-modifying therapies are currently available on the market for the treatment of Rett syndrome, we are aware of several companies that are in clinical or preclinical stages of developing gene therapies for the treatment of this disease. Taysha Gene Therapies, Inc. has a clinical-stage gene therapy program for the treatment of Rett syndrome. Stoke Therapeutics, Inc., in partnership with Acadia Pharmaceuticals Inc. (“Acadia”), and Alcyone Therapeutics, Inc. have disclosed the existence of early preclinical or discovery-stage gene therapy programs for the treatment of Rett syndrome. In July 2023, Acadia announced the acquisition of worldwide rights to NNZ-2591 for Rett syndrome, which is an investigational synthetic analogue of cyclo-glycyl-proline being developed in several neurodevelopmental syndromes.

DAYBUE (trofinetide) was approved by the FDA in March 2023 and by Health Canada in October 2024 and is a commercially available treatment in the U.S. and Canada from Acadia for the treatment of Rett syndrome in adults and pediatric patients two years and older. Additionally, Acadia has acquired ex-North American rights to trofinetide and has announced plans to seek approval for trofinetide for Europe, Asia and other regions in the future. However, we do not view trofinetide as directly competitive to our product candidate given the distinct mechanism of action of NGN-401, which we believe addresses the root cause of disease by replacing the missing protein.

The primary competitive factors that will affect the commercial success of any product candidate for which we may receive marketing approval include the efficacy, safety and tolerability profile, dosing convenience, price, coverage, reimbursement and public opinion. Some of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, as well as in obtaining regulatory approvals of those product candidates in the U.S. and in foreign countries. Some of our current and potential future competitors also have significantly more experience commercializing drugs that have been approved for marketing. Further, mergers, acquisitions and collaborations or partnerships in the biopharmaceutical industry could result in even more resources being concentrated among a small number of our competitors.

Accordingly, competitors may be more successful than us in obtaining regulatory approval for therapies and in achieving widespread market acceptance of their drugs. It is also possible that the development of a cure or more effective treatment method for any of our targeted indications by a competitor could render our product candidate non-competitive or obsolete, or reduce the demand for our product candidate before we can recover our development and commercialization expenses.

### **Government Regulation**

The FDA and other regulatory authorities at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring and post-approval reporting of biological products (“biologics”), such as those we are developing. We, along with our third-party contractors, will be required to navigate the various preclinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates. Generally, before a new therapeutic product can be marketed, considerable data demonstrating a biological product candidate’s quality, safety, purity and potency, or a small molecule drug candidate’s quality, safety and efficacy, must be obtained, organized into a format specific for each regulatory authority, submitted for review and approved by the regulatory authority. For biological product candidates, potency is similar to efficacy and is interpreted to mean the specific ability or capacity of the product, as indicated by appropriate laboratory tests or by adequately controlled clinical data obtained through the administration of the product in the manner intended, to effect a given result.

Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or post-marketing may subject an applicant to administrative or judicial sanctions. These sanctions could include, among other actions, the FDA's refusal to approve pending applications from the sponsor, withdrawal of an approval, a clinical hold, untitled or warning letters, product recalls or market withdrawals, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement and civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on our company and our products or product candidates.

### ***U.S. Biologics Regulation***

In the U.S., biological products like the product candidates we are developing are subject to regulation under the Federal Food, Drug, and Cosmetic Act ("FDCA") and the Public Health Service Act ("PHSA") and their implementing regulations, as well as other federal, state, local, and foreign statutes and regulations. Within the FDA, the Center for Biologics Evaluation and Research ("CBER") regulates biological products, including gene and cell therapies. CBER's Office of Therapeutic Products is responsible for oversight of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee advises CBER on its reviews. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or following approval may subject an applicant to administrative action and judicial sanctions. The process required by the FDA before biologic product candidates may be marketed in the U.S. generally involves the following:

- completion of preclinical laboratory tests and animal studies performed in accordance with applicable regulations, including the FDA's current Good Laboratory Practices;
- submission to the FDA of an IND, which must become effective before clinical trials may begin and to which annual reports and supplements or amendments for significant changes must be submitted;
- approval by an independent institutional review board ("IRB"), or ethics committee at each clinical site before the trial may be commenced;
- manufacture of the proposed biologic candidate in accordance with cGMPs, with methods and controls to ensure the product's identity, strength, quality, purity, safety and efficacy or potency;
- performance of adequate and well-controlled human clinical trials in accordance with applicable IND regulations, current Good Clinical Practice ("cGCP") requirements and other clinical-trial related regulations to establish the safety, purity and efficacy or potency of the proposed biologic product candidate for its intended purpose;
- preparation of and submission to the FDA of a biologics license application ("BLA"), after completion of pivotal clinical trials;
- a determination by the FDA within 60 days of its receipt of a BLA to file the application for review;
- satisfactory completion of an FDA Advisory Committee review, if applicable;
- satisfactory completion of an FDA pre-license inspection of the manufacturing facility or facilities at which the proposed product is produced to assess compliance with cGMPs, and to assure that the facilities, methods and controls are adequate to preserve the biological product's continued safety, purity and efficacy or potency, and potential audit of selected clinical investigation sites to assess compliance with cGCPs;
- FDA review and approval of a BLA to permit commercial marketing of the product for a particular indication(s) for use in the United States.
- payment of user fees under the Prescription Drug User Fee Act, unless waived;
- securing FDA approval of the BLA and licensure of the new biologic product; and



- compliance with any post-approval requirements, including, as applicable, the potential requirement to implement a Risk Evaluation and Mitigation Strategy (“REMS”), and any post-approval studies or other post-marketing commitments required by the FDA.

### ***Preclinical and Clinical Development***

Prior to beginning the first clinical trial with a product candidate, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol or protocols for clinical trials. The IND also includes results of animal and in vitro studies assessing the toxicology, pharmacokinetics, pharmacology and pharmacodynamic characteristics of the product (as applicable), chemistry, manufacturing and controls information, and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day period, raises safety concerns or questions about the proposed clinical trial. In such a case, the IND may be placed on a partial or full clinical hold. In the event of a clinical hold, the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND therefore may or may not result in FDA authorization to begin a clinical trial.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with cGCPs, which include the requirement that all research subjects provide their informed consent for their participation in a clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to an existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site, and must monitor the study until completed.

Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may recommend halting the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as the absence of efficacy. There are also requirements governing the reporting of ongoing preclinical studies and clinical trials and clinical study results to public registries. Sponsors of clinical trials of FDA-regulated products, including biological products, are required to register and disclose certain clinical trial information, which is publicly available at [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

A sponsor who wishes to conduct a clinical trial outside of the U.S. may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. If a foreign clinical trial is not conducted under an IND, the sponsor may submit data from the clinical trial to the FDA in support of a BLA. The FDA will accept a well-designed and well-conducted foreign clinical trial not conducted under an IND if the trial was conducted in accordance with cGCP requirements and the FDA is able to validate the data through an onsite inspection if deemed necessary.

For purposes of BLA approval, human clinical trials are typically conducted in three sequential phases that may overlap.

- *Phase 1.* The investigational product is initially introduced into healthy human subjects or patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.
- *Phase 2.* The investigational product is administered to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify

possible adverse side effects and safety risks. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.

- *Phase 3.* The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may be made a condition to approval of the BLA. Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the biological characteristics of the product candidate, and must finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product, or for biologics, the safety, purity and efficacy or potency. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data and clinical study investigators. Written IND safety reports must be promptly submitted to the FDA and the investigators for serious and unexpected suspected adverse reactions, any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information.

### ***BLA Submission and Review***

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, preclinical studies and clinical trials are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. FDA approval of a BLA must be obtained before a biologic may be marketed in the U.S. The BLA must include all relevant data available from pertinent preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls, and proposed labeling, among other things. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of the product, or from a number of alternative sources, including studies initiated and sponsored by investigators. The submission of a BLA requires payment of a substantial application user fee to the FDA, unless a waiver or exemption applies.

In addition, under the Pediatric Research Equity Act ("PREA"), a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biological product candidate for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The Food and Drug Administration Safety and Innovation Act requires that a sponsor who is planning to submit a marketing application for a biological product that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial pediatric study plan ("PSP") within sixty days after an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before the initiation of the Phase 3 or Phase 2/3 study as may be agreed between the sponsor and FDA. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and

the sponsor must reach an agreement on the PSP before initiation of pediatric studies. A sponsor can request amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs. Amendments should not be considered agreed upon until the FDA issues a letter stating that the amendments are acceptable. Unless otherwise required by regulation, PREA does not apply to any biological product for an indication for which orphan designation has been granted.

Within 60 days following submission of the BLA, the FDA reviews the application to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. Once a BLA has been accepted for filing, the FDA's goal is to review standard applications within ten months after the filing date, or, if the application qualifies for priority review, six months after the FDA accepts the application for filing. In both standard and priority reviews, the review process may also be extended by FDA requests for additional information or clarification. The FDA reviews a BLA to determine, among other things, whether a product is safe, pure and potent and the facility in which it is manufactured, processed, packed or held meets standards designed to assure the product's continued safety, purity and efficacy or potency. The FDA may convene an advisory committee to provide clinical insight on application review questions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving a BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with cGCPs. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it typically will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates a BLA and conducts inspections of manufacturing facilities where the product and/or its drug substance will be produced, the FDA may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for one or more specific indications. A Complete Response Letter will describe all of the deficiencies that the FDA has identified in the BLA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the Complete Response Letter without first conducting required inspections, testing submitted product lots and/or reviewing proposed labeling. In issuing a Complete Response Letter, the FDA may recommend actions that the applicant might take to place the BLA in condition for approval, including requests for additional information or clarification. If a Complete Response Letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application or request an opportunity for a hearing. The FDA may delay or refuse approval of a BLA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product.

If regulatory approval of a product is granted, such approval will be granted for one or more particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with a REMS to ensure the benefits of the product outweigh its risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a product and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as special training or monitoring requirements, restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once a BLA is approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. The FDA may require one or more Phase 4 post-market studies and surveillance to further assess and monitor the product's safety and

effectiveness after commercialization and may limit further marketing of the product based on the results of these post-marketing studies.

#### ***Additional Considerations for Gene Therapy Products***

In addition to the regulations discussed above, there are a number of additional considerations that apply to clinical trials involving the use of gene therapy. Supervision of human gene transfer trials includes evaluation and assessment by an institutional biosafety committee (“IBC”) a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. The FDA has issued various guidance documents regarding gene therapies, which outline additional factors that the FDA will consider at each of the above stages of development and relate to, among other things: the proper preclinical assessment of gene therapies; the CMC information that should be included in an IND application; the proper design of tests to measure product efficacy or potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. For instance, the FDA usually recommends that sponsors observe all surviving subjects who receive treatment using gene therapies that are based on adeno-associated virus vectors in clinical trials for potential gene therapy-related delayed adverse events for a minimum five-year period. FDA does not require the long-term tracking to be complete prior to its review of the BLA.

In 2024, the FDA began accepting applications from sponsors for the START pilot program with the purpose of further accelerating the pace of development of novel drug and biological products that are intended to address an unmet medical need as a treatment for a rare disease. The pilot is designed to be milestone-driven (i.e., to facilitate the progression of a development program to pivotal clinical study stage or the pre-BLA meeting stage) where product development programs selected would benefit from enhanced communications with FDA. In June 2024, we announced that NGN-401 for Rett syndrome was selected by FDA for the START pilot program.

A product candidate may be eligible for participation in the START Pilot program if an IND has been submitted in electronic common technical document format unless a waiver is granted; the sponsor has demonstrated substantial effort to ensure that CMC development aligns with clinical development; and, for CBER-regulated products, the product must be under development towards a marketing application under an existing OTP-regulated IND, and is intended to address an unmet medical need as a treatment for a rare disease or serious condition, which is likely to lead to significant disability or death within the first decade of life. The START Pilot Program is intended to provide a mechanism for addressing clinical development issues that otherwise would delay or prevent a promising novel drug or biological product from progressing to the pivotal clinical trial stage or pre-BLA meeting stage. Participants in the START Pilot Program will receive enhanced communications with FDA review staff. These enhanced communications will include, at a minimum, an initial meeting to review features of the pilot, discuss a pathway intended to support a marketing application, and discuss specific issues for which a sponsor requests enhanced communication with FDA. Additional communications will include ongoing interactions via email or teleconference that take place on a scheduled and/or as needed basis as agreed upon by the sponsor and FDA.

#### ***Expedited Development and Review Programs***

The FDA offers a number of expedited development and review programs for qualifying product candidates. The fast track program is intended to expedite or facilitate the process for reviewing new products that meet certain criteria. Specifically, new products are eligible for fast track designation if they are intended to treat a serious or life-threatening disease or condition and data demonstrate the potential to address unmet medical needs for the disease or condition. Fast track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a new biological product may request the FDA to designate the biological product as a Fast Track product at any time during the clinical development of the product. The sponsor of a fast track product has opportunities for more frequent interactions with the review team during product development and, once a BLA is submitted, the product may be eligible for priority review. A fast track product may also be eligible for rolling review, where the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA, the FDA

agrees to accept sections of the BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA. We have received fast track designation for NGN-401 for the treatment of Rett syndrome.

A product may also be eligible for breakthrough therapy designation to expedite its development and review if it is intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the fast track program features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product, including involvement of senior managers. We may seek breakthrough therapy designation for one or more of our product candidates, including NGN-401 for the treatment of Rett syndrome.

Regenerative medicines, which include AAV gene therapies like the one we are using in our NGN-401 clinical trial for the treatment of Rett syndrome, are eligible to receive the regenerative medicine advanced therapy (“RMAT”) designation. An RMAT is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions. Such a product is eligible for RMAT designation if it is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates it has the potential to address unmet medical needs for such disease or condition. Advantages of the RMAT designation include early interactions with the FDA to discuss the development plan for the product candidate, including potential surrogate or intermediate endpoints, and eligibility for rolling and priority review. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion to additional sites. RMAT-designated products that receive accelerated approval may, as appropriate, fulfill their post-approval requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real-world evidence (such as electronic health records); through the collection of larger confirmatory data sets; or via post-approval monitoring of all patients treated with such therapy prior to approval of the therapy. In August 2024, we announced the NGN-401 had received RMAT designation for the treatment of Rett syndrome. We may also request RMAT designation for future product candidates.

Any marketing application for a biologic submitted to the FDA for approval, including a product with a fast track designation, breakthrough therapy designation, START designation and/or RMAT designation, may be eligible for other types of FDA programs intended to expedite the FDA review and approval process, such as priority review and accelerated approval. A product is eligible for priority review if it has the potential to provide a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention of a serious disease or condition. For original BLAs, priority review designation means the FDA’s goal is to take action on the marketing application within six months of the 60-day filing date (as compared to ten months under a standard review). We have not sought priority review for any of our product candidates to date, but may do so in the future, including for NGN-401 for the treatment of Rett syndrome.

Additionally, products intended for use in treating serious or life-threatening diseases or conditions may receive accelerated approval upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA will generally require the sponsor to perform adequate and well-controlled post-marketing clinical studies with due diligence to verify and describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. Under the Food and Drug Omnibus Reform Act of 2022 (“FDORA”), the FDA may require, as appropriate, that such studies be underway prior to approval or within a specific time period after the date of approval for a product granted accelerated approval. Under FDORA, the FDA has increased authority for expedited procedures to withdraw approval of a product or indication approved under accelerated approval if the sponsor fails to conduct the required post-marketing studies or if such studies fail to verify the predicted clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval

pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

Fast track designation, breakthrough therapy designation, START designation, RMAT designation and priority review do not change the standards for approval but may expedite the development or approval process. Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

### ***Orphan Drug Designation and Exclusivity***

Under the Orphan Drug Act of 1983, the FDA may grant orphan drug designation to a product candidate intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the U.S., or 200,000 or more individuals in the U.S. for which there is no reasonable expectation that the cost of developing and making available in the U.S. a drug or biologic for this type of disease or condition will be recovered from sales in the U.S. for that product candidate. Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. We have received an orphan drug designation for NGN-401 for the treatment of Rett syndrome. The orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusive approval (or exclusivity), which means that the FDA may not approve any other applications, including a full BLA, to market the same product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity by means of greater effectiveness, greater safety or providing a major contribution to patient care or if the holder of the orphan drug exclusivity cannot assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the product was designated. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the BLA application fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan drug designation. In addition, exclusive marketing rights in the U.S. may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

There is some uncertainty with respect to the FDA's interpretation of the scope of orphan drug exclusivity. Historically, exclusivity was specific to the orphan indication for which the drug was approved. As a result, the scope of exclusivity was interpreted as preventing approval of a competing product. However, in 2021, the federal court in *Catalyst Pharmaceuticals, Inc. v. Becerra* suggested that orphan drug exclusivity covers the full scope of the orphan-designated "disease or condition" regardless of whether a drug obtained approval for a narrower use.

### ***Post-Approval Requirements***

Any products manufactured or distributed by us pursuant to FDA-approved applications are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and promotion of the product. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. After a BLA is approved for a biological product, the product also may be subject to official lot release. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, and potency or effectiveness of biologics. After approval, most changes to the

approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing user fee requirements, under which the FDA assesses an annual program fee for each product identified in an approved BLA. Biologic manufacturers like us and their subcontractors are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMPs, which impose certain procedural and documentation requirements upon such companies and their third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMPs and impose reporting requirements upon such companies and any third-party manufacturers that they may decide to use. Accordingly, we must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMPs and other aspects of regulatory compliance.

The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of a product, complete withdrawal of the product from the market or product recalls;
- fines, warning, untitled, or it has come to our attention letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of existing product approvals;
- product seizure or detention, or refusal of the FDA to permit the import or export of products;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of biologics. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by drug manufacturers and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

In addition, the distribution of prescription drug products, including most biological products that require a prescription, are subject to the Prescription Drug Marketing Act, or the PDMA, which regulates the distribution of drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription drug product samples and impose requirements to ensure accountability in distribution.

### ***Biosimilars and Reference Product Exclusivity***

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (collectively, the “ACA”), includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 (“BPCIA”), which created an abbreviated approval pathway for biological products that are highly similar, or “biosimilar,” to or interchangeable with an FDA-approved reference biological product. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars.

Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and efficacy or potency, is generally shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. A product shown to be biosimilar or interchangeable with an FDA-approved reference biological product may rely in part on the FDA’s previous determination of safety and effectiveness for the reference product for approval, which can potentially reduce the cost and time required to obtain approval to market the product. Complexities associated with the larger, and often more complex, structures of biological products, as well as the processes by which such products are manufactured, pose significant hurdles to implementation of the abbreviated approval pathway that are still being worked out by the FDA. In September 2021, the FDA issued two guidance documents intended to inform prospective applicants and facilitate the development of proposed biosimilars and interchangeable biosimilars, as well as to describe the FDA’s interpretation of certain statutory requirements added by the BPCIA.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing that applicant’s own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and efficacy or potency of its product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. FDA-approved interchangeable biosimilars may be substituted for the reference product without the intervention of the prescribing health care provider, subject to state laws, which differ by state.

A reference biologic is granted 12 years of exclusivity from the time of first licensure of the reference product. The first biologic product submitted under the abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against other biologics submitted under the abbreviated approval pathway for the lesser of (i) one year after the first commercial marketing, (ii) 18 months after approval if there is no legal challenge, (iii) 18 months after the resolution in the applicant’s favor of a lawsuit challenging the biologics’ patents if an application has been submitted, or (iv) 42 months after the application has been approved if a lawsuit is ongoing within the 42-month period.

A biological product can also obtain pediatric market exclusivity in the U.S. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued “Written Request” for such a study.

The BPCIA is complex and continues to be interpreted and implemented by the FDA. In July 2018, the FDA announced an action plan to encourage the development and efficient review of biosimilars, including the establishment of a new office within the agency that will focus on therapeutic biologics and biosimilars. On December 20, 2020, Congress amended the PHSA as part of the COVID-19 relief bill to further simplify the biosimilar review process by making it optional to show that conditions of use proposed in labeling have been previously approved for the reference product, which used to be a requirement of the application. In addition, government proposals have sought to reduce the 12-year reference product exclusivity period. Other aspects of the



BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. As a result, the ultimate impact, implementation, and impact of the BPCIA is subject to significant uncertainty.

As discussed below, the Inflation Reduction Act of 2022 (“IRA”) is a significant new law that intends to foster generic and biosimilar competition and to lower drug and biologic costs.

#### ***Patent Term Extension***

In the U.S., after a BLA is approved, owners of relevant drug patents may apply for up to a five-year patent extension, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory process. The allowable patent term extension is typically calculated as one-half the time between, the latter of the effective date of an IND and issue date of the patent for which extension is sought, and the submission date of a BLA, plus the time between BLA submission date and the BLA approval date up to a maximum of five years. The time can be shortened if the FDA determines that the applicant did not pursue licensure with due diligence. The total patent term after the extension may not exceed 14 years from the date of product licensure. Only one patent applicable to a licensed biological product is eligible for extension and only those claims covering the product, a method for using it, or a method for manufacturing it may be extended and the application for the extension must be submitted prior to the expiration of the patent in question. However, Neurogene may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Some, but not all, foreign jurisdictions possess patent term extension or other additional patent exclusivity mechanisms that may be more or less stringent and comprehensive than those of the U.S.

#### ***Rare Pediatric Disease Designation and Priority Review Vouchers***

Under the FDCA, as amended, the FDA incentivizes the development of drugs and biologics intended to treat conditions that meet the definition of a “rare pediatric disease,” defined to mean a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and the disease affects fewer than 200,000 individuals in the U.S. or affects more than 200,000 in the U.S. and for which there is no reasonable expectation that the cost of developing and making in the U.S. a drug for such disease or condition will be recovered from sales in the U.S. of such drug. Rett syndrome qualifies as a rare pediatric disease, and we have received rare pediatric disease designation for NGN-401 for the treatment of Rett syndrome, and we may request such designation for future product candidates if the diseases they are intended to treat meet the definition of a rare pediatric disease. The sponsor of a product candidate for a rare pediatric disease may be eligible for a voucher that can be used to obtain a priority review for a subsequent human drug or biologic application after the date of approval of the rare pediatric disease drug product, referred to as a priority review voucher (a “PRV”). A sponsor may request rare pediatric disease designation from the FDA prior to the submission of its BLA. A rare pediatric disease designation does not guarantee that a sponsor will receive a PRV upon approval of its BLA. Moreover, a sponsor who chooses not to submit a rare pediatric disease designation request may nonetheless receive a PRV upon approval of their marketing application if they request such a voucher in their original marketing application and meet all of the eligibility criteria. If a PRV is received, it may be sold or transferred an unlimited number of times. Congress has extended the PRV program to allow for the designation of drugs for rare pediatric diseases through December 20, 2024, with the potential for PRVs to be granted through September 30, 2026. If Congress does not further extend this program, we may not meet the deadline for PRVs to be granted for our current programs given the expected timeline of development.

#### ***Other Healthcare Laws and Compliance Requirements***

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business. Such laws include, without limitation: the federal Anti-Kickback Statute (“AKS”); the federal False Claims Act (“FCA”); the federal Civil Monetary Penalties Law (“CMPL”); the Health Insurance Portability and Accountability Act of 1996 (“HIPAA”); and similar foreign, federal and state fraud, abuse and transparency laws and regulations.

The AKS prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, to induce, or in return for, either the referral of an individual, or the purchase, lease, order, arrangement, or recommendation of an item or service for which payment may be made under any federal healthcare program. The term remuneration has been interpreted broadly to include anything of value. The AKS has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand, and prescribers and purchasers on the other. The government often takes the position that to violate the AKS, only one purpose of the remuneration need be to induce referrals, even if there are other legitimate purposes for the remuneration. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from AKS liability, but they are drawn narrowly and practices that involve remuneration, such as consulting agreements, may be subject to scrutiny if they do not qualify for an exception or safe harbor. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor. Failure to meet all of the requirements of a particular statutory exception or regulatory safe harbor does not make the conduct per se illegal under the AKS. Instead, the legality of a given arrangement is a fact-specific determination. Violations of the AKS are subject to civil and criminal fines and penalties for each violation, plus up to three times the remuneration involved, imprisonment, and exclusion from government healthcare programs. In addition, the government may assert that a claim including items or services resulting from a violation of the AKS constitutes a false or fraudulent claim for purposes of the FCA or a violation of the CMPL.

Civil and criminal false claims laws, including the FCA, and civil monetary penalty laws, which impose criminal and civil penalties and can be enforced through civil whistleblower or qui tam actions, prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment of federal government funds, including federal healthcare program funds, that are false or fraudulent; knowingly making, using or causing to be made or used, a false statement or record material to a false or fraudulent claim for payment or to an obligation to pay or transmit money or property to the federal government, or knowingly concealing or knowingly and improperly avoiding or decreasing an obligation to pay money to the federal government. Pharmaceutical and other healthcare companies have been prosecuted under these laws for engaging in a variety of different types of conduct that “caused” the submission of false claims to federal healthcare programs. Under the AKS, for example, a claim resulting from a violation of the AKS is deemed to be a false or fraudulent claim for purposes of the FCA. The FCA imposes mandatory treble damages and per-violation civil penalties up to approximately \$28,000. The FCA also permits a private individual acting as a “relator” to bring actions on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery, as well as to obtain attorneys’ fees and costs.

The CMPL imposes administrative sanctions for, among other things, providing remuneration to government health care program beneficiaries to influence them to order or receive health care items or services, and presenting or causing to be presented false claims for government payment.

HIPAA created additional federal criminal statutes that prohibit, among other things, executing a scheme to defraud any healthcare benefit program, including private third-party payors, and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements or representations relating to healthcare matters.

The FDCA addresses, among other things, the design, production, labeling, promotion, manufacturing, and testing of drugs, biologics and medical devices, and prohibits such acts as the introduction into interstate commerce of adulterated or misbranded drugs or devices. The PHSA also prohibits the introduction into interstate commerce of unlicensed or mislabeled biological products.

The U.S. federal Physician Payments Sunshine Act (the “Physician Payments Sunshine Act”) requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children’s Health Insurance Program, with specific exceptions, to annually report to the Centers for Medicaid & Medicare Services (“CMS”) information related to payments or other transfers of value to various healthcare professionals including physicians, certain other licensed health care practitioners, and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Beginning on January 1, 2023, California Assembly Bill 1278 requires California physicians and surgeons to notify patients of the Open Payments database established under the Physician Payments Sunshine Act.

We are also subject to federal price reporting laws and federal consumer protection and unfair competition laws. Federal price reporting laws require manufacturers to calculate and report complex pricing metrics to government programs, where such reported prices may be used in the calculation of reimbursement and/or discounts on approved products. Federal consumer protection and unfair competition laws broadly regulate marketplace activities and activities that potentially harm consumers.

Further, we are subject to additional similar U.S. state and foreign law equivalents of each of the above federal laws, which, in some cases, differ from each other in significant ways, and may not have the same effect, thus complicating compliance efforts. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply, we may be subject to penalties, including, without limitation, civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations.

### ***Data Privacy and Security***

Numerous state, federal, and foreign laws govern the collection, dissemination, use, access to, confidentiality, and security of personal information, including health-related information. In the U.S., numerous federal and state laws and regulations, including state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws and regulations that govern the collection, use, disclosure, and protection of health-related and other personal information apply to our operations or the operations of our partners. For example, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health (“HITECH”), and their respective implementing regulations impose privacy, security, and breach notification obligations on certain health care providers, health plans, and health care clearinghouses, known as covered entities, as well as their business associates and subcontractors that perform services on their behalf that involve using, disclosing, creating, receiving, maintaining, or transmitting individually identifiable health information for or on behalf of such covered entities. The requirements imposed by HIPAA and HITECH on covered entities and business associates include entering into agreements that require business associates protect protected health information (“PHI”) provided by the covered entity against improper use or disclosure, among other things; following certain standards for the privacy of PHI, which limit the disclosure of a patient’s past, present or future physical or mental health or condition or information about a patient’s receipt of health care if the information identifies, or could reasonably be used to identify, the individual; ensuring the confidentiality, integrity and availability of all PHI created, received, maintained or transmitted in electronic form, in particular in order to identify and protect against reasonably anticipated threats or impermissible uses or disclosures to the security and integrity of such PHI; and reporting of such breaches of PHI to individuals, the U.S. Department of Health and Human Services (“HHS”) and, in certain circumstances, the media.

Significant civil and criminal fines and other penalties may be imposed for violating HIPAA. A covered entity or business associate is also liable for civil money penalties for a violation that is based on an act or omission of any of its agents, which may include a downstream business associate, as determined according to the federal common law of agency. HITECH also increased the civil and criminal penalties applicable to covered entities and business associates and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorneys’ fees and costs associated with pursuing federal civil actions. To the extent that we submit electronic healthcare claims and payment transactions that do not comply with the electronic data transmission standards established under HIPAA and HITECH, payments to us may be delayed or denied.

Even when HIPAA does not apply, according to the FTC, violating consumers’ privacy rights or failing to take appropriate steps to keep consumers’ personal information secure may constitute unfair acts or practices in or affecting commerce in violation of Section 5(a) of the Federal Trade Commission Act. Many state consumer protection laws similarly provide state-law causes of action for allegedly unfair or deceptive acts or practices, among other things, including causes of action for alleged data privacy violations.

In addition, certain state laws, such as the California Consumer Privacy Act of 2018 (“CCPA”), as amended by the California Privacy Rights Act of 2020 (“CPRA”), California’s Confidentiality of Medical Information Act, and

Washington's My Health My Data Act govern the privacy and security of personal information, including health-related information in certain circumstances, some of which are more stringent than HIPAA and many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. The CCPA/CPRA applies to personal data of California consumers, including business representatives and employees, and imposes obligations on certain businesses that do business in California, including to provide specific disclosures in privacy notices, and affords certain rights to California residents in relation to their personal information. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and/or criminal penalties and, in some cases, private litigation, as well as injunctive restrictions on data processing. Privacy and security laws, regulations, and other obligations are constantly evolving, may conflict with each other, which complicates compliance efforts, and can result in investigations, proceedings, or actions that lead to significant civil and/or criminal penalties and restrictions on data processing. Additionally, our use of artificial intelligence and machine learning may be subject to laws and evolving regulations regarding the use of artificial intelligence/machine learning, controlling for data bias, and anti-discrimination.

### ***Coverage and Reimbursement***

In the U.S. and markets in other countries, patients generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. Our ability to successfully commercialize our product candidates will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow it to establish or maintain pricing sufficient to realize a sufficient return on its investment. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels.

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical or biological product for which we may obtain regulatory approval. Sales of any product, if approved, depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state, and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement, if any, for such product by third-party payors. Decisions regarding whether to cover any of its product candidates, if approved, the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. Further, no uniform policy for coverage and reimbursement exists in the U.S., and coverage and reimbursement can differ significantly from payor to payor. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. As a result, the coverage determination process is often a time-consuming and costly process that will require it to provide scientific and clinical support for the use of its product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Factors payors consider in determining reimbursement are based on whether the product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost effectiveness of pharmaceutical or biological products, medical devices and medical services, in addition to questioning safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could

further limit sales of any product that receives approval. Decreases in third-party reimbursement for any product or a decision by a third-party not to cover a product could reduce physician usage and patient demand for the product.

For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization. In addition, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics.

In addition, net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the U.S. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product candidate that we may commercialize and, if reimbursement is available, the level of reimbursement. In addition, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average sales price, or ASP, and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely. Further, these prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs.

Finally, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost effectiveness of a particular product candidate to currently available therapies. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our product candidates. Historically, products launched in the European Union do not follow price structures of the U.S. and generally prices tend to be significantly lower.

### ***Healthcare Reform***

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the U.S., the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by federal and state legislative initiatives, including those designed to limit the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded health care programs, and increased governmental control of drug pricing.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (“ACA”), which was enacted in 2010, substantially changed the way healthcare is financed by both governmental and private insurers in the U.S., and significantly affected the pharmaceutical industry. The ACA contains a number of provisions of particular import to the pharmaceutical and biotechnology industries, including, but not limited to, those governing enrollment in federal healthcare programs. Since its enactment, there have been judicial and Congressional challenges to certain aspects of the ACA, and Neurogene expects there will be additional challenges and amendments to the ACA in the future.

Other legislative changes have been proposed and adopted since the ACA was enacted. For example, the Budget Control Act of 2011 and subsequent legislation, among other things, created measures for spending

reductions by Congress that include aggregate reductions of Medicare payments to providers of 2% per fiscal year, which remain in effect through 2032. Due to the Statutory Pay-As-You-Go Act of 2010, estimated budget deficit increases resulting from the American Rescue Plan Act of 2021, and subsequent legislation, Medicare payments to providers will be further reduced starting in 2025 absent further legislation. The U.S. American Taxpayer Relief Act of 2012 further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

In addition, the Bipartisan Budget Act of 2018, among other things, amended the Medicare Act (as amended by the ACA) to increase the point-of-sale discounts that manufacturers must agree to offer under the Medicare Part D coverage discount program to 70% off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs being covered under Medicare Part D.

Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state measures designed to, among other things, reduce the cost of prescription drugs, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. For example, in May 2019, CMS adopted a final rule allowing Medicare Advantage Plans the option to use step therapy for Part B drugs, permitting Medicare Part D plans to apply certain utilization controls to new starts of five of the six protected class drugs, and requiring the Explanation of Benefits for Part D beneficiaries to disclose drug price increases and lower cost therapeutic alternatives.

In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. The IRA includes several provisions that may impact our business to varying degrees, including provisions that reduce the out-of-pocket spending cap for Medicare Part D beneficiaries from \$7,050 to \$2,000 starting in 2025, thereby effectively eliminating the coverage gap; impose new manufacturer financial liability on certain drugs under Medicare Part D, allow the U.S. government to negotiate Medicare Part B and Part D price caps for certain high-cost drugs and biologics without generic or biosimilar competition; require companies to pay rebates to Medicare for certain drug prices that increase faster than inflation; and delay until January 1, 2032 the implementation of the HHS rebate rule that would have limited the fees that pharmacy benefit managers can charge. Further, under the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one rare disease designation and for which the only approved indication is for that disease or condition. If a product receives multiple rare disease designations or has multiple approved indications, it may not qualify for the orphan drug exemption. The effects of the IRA on its business and the healthcare industry in general is not yet known.

President Biden has also issued multiple executive orders that have sought to reduce prescription drug costs. In February 2023, HHS also issued a proposal in response to an October 2022 executive order from President Biden that includes a proposed prescription drug pricing model that will test whether targeted Medicare payment adjustments will sufficiently incentivize manufacturers to complete confirmatory trials for drugs approved through FDA's accelerated approval pathway. Although a number of these and other proposed measures may require authorization through additional legislation to become effective, and the Biden administration may reverse or otherwise change these measures, both the Biden administration and Congress have indicated that they will continue to seek new legislative measures to control drug costs.

Notwithstanding the IRA and President Biden's executive orders, continued legislative and enforcement interest exists in the U.S. with respect to specialty drug pricing practices. Specifically, we expect regulators to continue pushing for transparency to drug pricing, reducing the cost of prescription drugs under Medicare, reviewing the relationship between pricing and manufacturer patient programs, and reforming government program reimbursement methodologies for drugs.

Individual states in the U.S. have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient

reimbursement constraints, discounts, restrictions on certain drug access and marketing cost disclosure and transparency measures, and designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, financial condition, results of operations and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for its drugs or put pressure on its drug pricing, which could negatively affect our business, financial condition, results of operations and prospects.

#### ***Other Government Regulation Outside of the United States***

In addition to regulations in the U.S., we are subject to a variety of regulations in other jurisdictions governing, among other things, research and development, clinical trials, testing, manufacturing, safety, efficacy, quality control, labeling, packaging, storage, record keeping, distribution, reporting, export and import, advertising, marketing and other promotional practices involving biological products as well as authorization, approval as well as post-approval monitoring and reporting of its products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the U.S. have a similar process that requires the submission of a clinical trial application much like an IND prior to the commencement of human clinical trials.

The requirements and process governing the conduct of clinical trials, including requirements to conduct additional clinical trials, product licensing, safety reporting, post-authorization requirements, marketing and promotion, interactions with healthcare professionals, pricing and reimbursement may vary widely from country to country. No action can be taken to market any product in a country until an appropriate approval application has been approved by the regulatory authorities in that country. The current approval process varies from country to country, and the time spent in gaining approval varies from that required for FDA approval. In certain countries, the sales price of a product must also be approved. The pricing review period often begins after market approval is granted. Even if a product is approved by a regulatory authority, satisfactory prices may not be approved for such product, which would make launch of such products commercially unfeasible in such countries.

#### ***Regulation in the European Union***

##### *EEA/UK Data Protection Laws*

The processing, including the collection and use of personal health data and other personal data regarding individuals in the European Economic Area (“EEA”) is governed by the provisions of the European General Data Protection Regulation 2016/679 (“EU GDPR”) and related data protection laws in individual EEA member states, including additional requirements relating to health, genetic and biometric data implemented through national legislation. Similarly, processing of personal health data and other personal data regarding individuals in the UK is governed by the UK General Data Protection Regulation (“UK GDPR”) and the UK Data Protection Act 2018. In this document, “GDPR” refers to both the EU GDPR and the UK GDPR, unless specified otherwise. The GDPR imposes a number of strict obligations and restrictions on the ability to process, including collecting, analyzing and transferring, personal data of individuals, in particular with respect to health data and personal data breach reporting. The GDPR includes requirements relating to the legal basis of the processing (such as consent of the individuals to whom the personal data relates), the information provided to the individuals prior to processing their personal data, the notification obligations to the national data protection authorities and individuals, and the security and confidentiality of personal data. Because we are conducting a portion of our ongoing clinical trials in the UK, these requirements will apply to us with respect to the data collected from individuals participating in those trials in the UK.

In addition, the GDPR imposes specific restrictions on the transfer of personal data to countries outside of the EEA/UK that are not considered by the European Commission (“EC”) and the UK government as providing an adequate level of data protection. Appropriate safeguards are required to enable such transfers. Among the

appropriate safeguards that can be used, the data exporter may use the EC approved standard contractual clauses (“SCCs”) adopted in 2021 and the UK International Data Transfer Agreement/Addendum (“UK IDTA”) adopted in 2022. Where relying on the SCCs and/or UK IDTA for data transfers, we are also required to carry out transfer impact assessments to verify if anything in the law and/or practices of the third country may impinge on the effectiveness of the SCCs and/or UK IDTA in the context of the transfer at stake and, if so, to identify and adopt supplementary measures. Where no supplementary measure is suitable, the data exporter should avoid, suspend or terminate the transfer. Although the UK is regarded as a third country under the EU GDPR, the EC has issued a decision recognizing the UK as providing adequate protection under the EU GDPR (“Adequacy Decision”) and, therefore, transfers of personal data originating in the EEA to the UK remain unrestricted. However, the Adequacy Decision include a ‘sunset clause’ which entails that the decision will automatically expire four years after its entry into force, i.e., on June 27, 2025, unless renewed.

With regard to the transfer of data from the EEA to the U.S., on July 10, 2023, the EC adopted its adequacy decision for the EU-US Data Privacy Framework. On the bases of the new adequacy decision, personal data can flow from the EEA to US companies participating in the framework. With regard to the transfer of personal data from the UK to the U.S., the UK government has adopted an adequacy decision for the U.S. (the “UK-US Data Bridge”), which came into force on October 12, 2023. The UK-US Data Bridge recognizes the U.S. as offering an adequate level of data protection where the transfer is to a U.S. company participating in the EU-US Data Privacy Framework and the UK Extension to the EU-US Data Privacy Framework.

The UK government has introduced a Data Protection and Digital Information Bill (“UK Data Protection Bill”) into the UK legislative process with the intention for this bill to reform the UK’s data protection regime following the UK’s succession from the EU. If passed, the final version of the UK Data Protection Bill may have the effect of further altering the similarities between the UK and EU data protection regime. This may lead to additional compliance costs and could increase our overall risk. The respective provisions and enforcement of the EU GDPR and UK GDPR may further diverge in the future and create additional regulatory challenges and uncertainties.

Failure to comply with the requirements of the GDPR and the related national data protection laws of the EEA member states/UK may result in significant monetary fines for noncompliance of up to €20 million (£17.5 million for the UK) or 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, other administrative penalties and a number of criminal offenses (punishable by uncapped fines) for organizations and, in certain cases, their directors and officers, as well as civil liability claims from individuals whose personal data was processed. Data protection authorities from the different EEA member states/UK may still implement certain variations, enforce the GDPR and national data protection laws differently, and introduce additional national regulations and guidelines, which adds to the complexity of processing personal data subject to the EEA/UK data protection regimes. Guidance developed at both the EU level and at the national level in individual EU member states concerning implementation and compliance practices are often updated or otherwise revised.

Compliance with the GDPR is a rigorous and time-intensive process that may increase our cost of doing business or require us to change our business practices, and despite those efforts, there is a risk that we may be subject to fines, penalties and litigation in connection with EEA/UK, which could in turn have a negative effect on our reputation and materially harm our business.

Furthermore, there is a growing trend towards the required public disclosure of clinical trial data in the EU, which adds to the complexity of obligations relating to processing health data from clinical trials. Such public disclosure obligations are provided in the EU Clinical Trials Regulation (EU) No. 536/2014 (the “CTR”), EMA disclosure initiatives and voluntary commitments by industry. Failure to comply with these obligations could lead to government enforcement actions and significant penalties against it, harm to its reputation, and adversely impact its business and operating results. The uncertainty regarding the interplay between different regulatory frameworks, such as the CTR and the GDPR, further adds to the complexity that we face with regard to data protection regulation.



### ***Drug and Biologic Development Process***

Regardless of where they are conducted, all clinical trials included in applications for marketing authorization for human medicines in the EU must have been carried out in accordance with EU regulations. This means that clinical trials conducted in the EU have to comply with EU clinical trial legislation but also that clinical trials conducted outside the EU have to comply with ethical principles equivalent to those set out in the EU, including adhering to international good clinical practice and the Declaration of Helsinki. The conduct of clinical trials in the EU is governed by the CTR, which entered into force on January 31, 2022. The CTR replaced the Clinical Trials Directive 2001/20/EC, (“Clinical Trials Directive”) and introduced a complete overhaul of the existing regulation of clinical trials for medicinal products in the EU.

Under the former regime, which will expire after a transition period of one or three years from the date the CTR came into effect, respectively, as outlined below in more detail, before a clinical trial can be initiated it must be approved in each EU member state where there is a site at which the clinical trial is to be conducted. The approval must be obtained from two separate entities: the national competent authority in the applicable EU member state(s) and one or more ethics committees. The national competent authority of all EU member states in which the clinical trial will be conducted must authorize the conduct of the trial, and the independent ethics committee must grant a positive opinion in relation to the conduct of the clinical trial in the relevant EU member state before the commencement of the trial. Any substantial changes to the trial protocol or other information submitted with the clinical trial applications must be submitted to or approved by the relevant national competent authorities and ethics committees. Under the current regime all suspected unexpected serious adverse reactions to the investigated drug that occur during the clinical trial must be reported to the national competent authority and to the ethics committees of the EU member state where they occur.

A more unified procedure applies under the CTR. A sponsor can submit a single application for approval of a clinical trial through a centralized EU clinical trials portal (the “Clinical Trials Information System” or “CTIS”). One national competent authority (the reporting EU member state proposed by the applicant) will take the lead in validating and evaluating the application, and will consult and coordinate with the other concerned EU member states. If an application is rejected, it may be amended and resubmitted through the EU clinical trials portal. If an approval is issued, the sponsor may start the clinical trial in all concerned EU member states. However, a concerned EU member state may in limited circumstances declare an “opt-out” from an approval and prevent the clinical trial from being conducted in such member state. The CTR also aims to streamline and simplify the rules on safety reporting, and introduces enhanced transparency requirements such as mandatory submission of a summary of the clinical trial results to the EU database. The CTR foresees a three-year transition period from the date the CTR went into effect. Since January 31, 2023, submission of initial clinical trial applications via CTIS is mandatory and CTIS serves as the single entry point for submission of clinical trial-related information and data. By January 31, 2025, all ongoing trials approved under the former Clinical Trials Directive will need to comply with the CTR and have to be transitioned to CTIS. On July 19, 2023, the European Commission published guidance concerning the steps to be taken in this transition. This guidance provides, among other things, that (i) documentation which was previously assessed will not be reassessed, (ii) templates that were developed and endorsed by the EU Clinical Trials Expert Group to provide compliance with the CTR do not need to be updated and (iii) there is no need to retrospectively create a site suitability form, which are only necessary for new trial sites.

Under both the former regime and the CTR, national laws, regulations, and the applicable GCP and GLP standards must also be respected during the conduct of the trials, including the International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use guidelines on Good Clinical Practice and the ethical principles that have their origin in the Declaration of Helsinki.

During the development of a medicinal product, the EMA and national regulators within the EU provide the opportunity for dialogue and guidance on the development program. At the EMA level, this is usually done in the form of scientific advice, which is given by the Committee for Medicinal Products for Human Use (“CHMP”) on the recommendation of the Scientific Advice Working Party. A fee is incurred with each scientific advice procedure but is significantly reduced for designated orphan medicines. Advice from the EMA is typically provided based on questions concerning, for example, quality (chemistry, manufacturing and controls testing), nonclinical testing and

clinical studies, and pharmacovigilance plans and risk-management programs. Advice is not legally binding with regard to any future marketing authorization application (“MAA”) for the product concerned.

### ***Drug Marketing Authorization***

In the EU, medicinal products are subject to extensive pre- and post-market regulation by regulatory authorities at both the EU and national levels. To obtain regulatory approval of a product under the EU regulatory systems, we must submit an MAA under either the EU centralized procedure, or one of the national procedures in the EU.

To be used or sold in the UK, a drug must have an effective marketing authorization obtained by a centralized application through EMA or a national application. National applications are governed by the Human Medicines Regulations (SI 2012/1916) (the “HMRs”). Applications are made electronically through the UK’s MHRA Submissions Portal. The process from application to authorizations generally takes up to 210 days, excluding time taken to provide any additional information or data required by the MHRA.

On August 30, 2023, the MHRA published detailed guidance on its recently announced new International Reliance Procedure (“IRP”) for MAAs. The IRP applies since January 1, 2024, and replaces existing EU reliance procedures to apply for authorizations from 7 international regulators (e.g. Health Canada, Swiss Medic, FDA, EMA, among others). The IRP allows medicinal products approved in other jurisdictions that meet certain criteria to undergo a fast-tracked MHRA review to obtain and/or update a marketing authorization in the UK.

Applicants can submit initial MAAs to the IRP but the procedure can also be used throughout the lifecycle of a product for post-authorization procedures including line extensions, variations and renewals. Any authorization which is not followed by the actual placing of the drug on the EU market (in case of centralized procedure) or on the market of the authorizing member state within three years after authorization ceases to be valid.

### ***Centralized Authorization Procedure***

The centralized procedure provides for the grant of a single marketing authorization (“MA”) that is issued by the EC following the scientific assessment of the application by the EMA and that is valid for all EU member states as well as in the three additional EEA member states (Norway, Iceland and Liechtenstein). The centralized procedure is compulsory for certain types of medicinal products, including for medicines developed by means of certain biotechnological processes, products designated as orphan medicinal products, advanced therapy medicinal products (gene therapy, somatic cell therapy or tissue-engineered medicines) and medicinal products with a new active substance indicated for the treatment of certain diseases (HIV/AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and other immune dysfunctions and viral diseases). The centralized procedure is optional for medicinal products containing a new active substance not yet authorized in the European Economic Area before May 20, 2004 and indicated for the treatment of other diseases, or for products that constitute a significant therapeutic, scientific or technical innovation or for which the grant of an MA through the centralized procedure would be in the interest of public health at EU level.

Under the centralized procedure, the CHMP established at the EMA, is responsible for conducting the initial assessment of a drug. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. Under the centralized procedure, the timeframe for the evaluation of an MAA by the EMA’s CHMP is, in principle, 210 days from receipt of a valid MAA. However, this timeline excludes clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP, so the overall process typically takes a year or more, unless the application is eligible for an accelerated assessment. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, particularly from the point of view of therapeutic innovation.

Upon request, the CHMP can reduce the time frame to 150 days if the applicant provides sufficient justification for an accelerated assessment. The CHMP will provide a positive opinion regarding the application only if it meets certain quality, safety and efficacy requirements. This opinion is then transmitted to the EC, which has the ultimate authority for granting the MA within 67 days after receipt of the CHMP opinion.

### ***Decentralized and Mutual Recognition Procedures***

Medicines that fall outside the mandatory scope of the centralized procedure can be authorized under a decentralized procedure where an applicant applies for simultaneous authorization in more than one EU member state, or they can be authorized in an EU member state in accordance with that state's national procedures and then be authorized in other EU countries by a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization (mutual recognition procedure).

The decentralized procedure permits companies to file identical MA applications for a medicinal product to the competent authorities in various EU member states simultaneously if such medicinal product has not received marketing approval in any EU member state before. The competent authority of a single EU member state, the reference member state, is appointed to review the application and provide an assessment report. The competent authorities of the other EU member states, the concerned member states, are subsequently required to grant a marketing authorization for their territories on the basis of this assessment. The only exception to this is where the competent authority of an EU member state considers that there are concerns of potential serious risk to public health, the disputed points are subject to a dispute resolution mechanism and may eventually be referred to the EC, whose decision is binding for all EU member states.

### ***Risk Management Plan***

All new MAAs must include a Risk Management Plan ("RMP") describing the risk management system that the company will put in place and documenting measures to prevent or minimize the risks associated with the product. RMPs are continually modified and updated throughout the lifetime of the medicine as new information becomes available. An updated RMP must be submitted: (i) at the request of EMA or a national competent authority, or (ii) whenever the risk-management system is modified, especially as the result of new information being received that may lead to a significant change to the benefit-risk profile or as a result of an important pharmacovigilance or risk-minimization milestone being reached. The regulatory authorities may also impose specific obligations as a condition of the MA. Since October 20, 2023, all RMPs for centrally authorized products are published by the EMA, subject to only limited redactions.

### ***MA Validity Period***

In the EU, an MA has an initial duration of five years. After these five years, the authorization may subsequently be renewed on the basis of a reevaluation of the risk-benefit balance. Once renewed, the MA is valid for an unlimited period unless the EC or the national competent authority decides on justified grounds relating to pharmacovigilance, to proceed with only one additional five-year renewal. Applications for renewal must be made to the EMA at least nine months before the five-year period expires.

Any authorization which is not followed by the actual placing of the drug on the EU market (in case of centralized procedure) or on the market of the authorizing member state within three years after authorization ceases to be valid.

For the UK, the period of three years during which the drug has not been marketed in Great Britain will be restarted from the date of conversion to a Great Britain marketing authorization. Conversion refers to the procedure by which, as of January 1, 2021, MAs granted on the basis of a centralized procedure in the EU are only valid in Northern Ireland but not in Great Britain, whereas, prior EU authorizations have all been automatically converted into UK MAs effective in Great Britain only.

On the other hand, for the EU, in case the drug has been marketed in the UK, the placing on the UK market before the end of the period starting when the UK left the EU on January 31, 2020, and ending on December 31, 2020 (the Brexit Transition Period) will be taken into account. If, after the end of the Brexit Transition Period, the drug is not placed on any other market of the remaining member states of the EU, the three-year period will start running from the last date the drug was placed on the UK market before the end of the Brexit Transition Period.

### ***Exceptional Circumstances/Conditional Approval***

Similar to accelerated approval regulations in the U.S., conditional MAs can be granted in the EU for medicines intended for treating, preventing or diagnosing seriously debilitating or life-threatening diseases, or in a public health emergency. A conditional MA can be granted for medicinal products where, although comprehensive clinical data referring to the safety and efficacy of the medicinal product have not been supplied, the following criteria are fulfilled: (i) the benefit/risk balance of the product is positive, (ii) it is likely that the applicant will be in a position to provide the comprehensive clinical data post-authorization, (iii) unmet medical needs will be fulfilled by the grant of the MA and (iv) the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. Once a conditional MA has been granted, the MA holder must fulfil specific obligations within defined timelines. A conditional MA is valid for one year and must be renewed annually, but it can be converted into a standard MA once the MA holder fulfils the obligations imposed and the complete data confirm that the medicine's benefits continue to outweigh its risks.

### ***Data and Market Exclusivity***

As in the U.S., it may be possible to obtain a period of market and/or data exclusivity in the EU that would have the effect of postponing the entry into the marketplace of a competitor's generic, hybrid or biosimilar product (even if the pharmaceutical product has already received a MA) and prohibiting another applicant from relying on the MA holder's pharmacological, toxicological and clinical data in support of another MA for the purposes of submitting an application, obtaining an MA or placing the product on the market. Innovative medicinal products (sometimes referred to as New Chemical Entities, "NCE") approved in the EU generally qualify for eight years of data exclusivity and 10 years of marketing exclusivity.

If granted, the data exclusivity period begins on the date of the product's first MA in the EU and prevents generic or biosimilar applicants from referencing the innovator's preclinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar marketing authorization in the EU. After eight years, a generic product application may be submitted and generic companies may rely on the MA holder's data. However, a generic product cannot launch until two years later (or a total of 10 years after the first MA in the EU of the innovator product). An additional one-year period of marketing exclusivity is possible if, during the data exclusivity period (the first eight years of the 10-year marketing exclusivity period), the MA holder obtains an MA for one or more new therapeutic indications that are deemed to bring a significant clinical benefit compared to existing therapies. Additionally, a standalone one-year period of data exclusivity can be granted where an application is made for a new indication for a well-established substance, provided that significant pre-clinical or clinical studies were carried out in relation to the new indication. Where a change of classification of a pharmaceutical product has been authorized on the basis of significant pre-trial tests or clinical trials, when examining an application by another applicant for or holder of an MA for a change of classification of the same substance the competent authority will not refer to the results of those tests or trials for one year after the initial change was authorized.

Products may not be granted data exclusivity since there is no guarantee that a product will be considered by the European Union's regulatory authorities to include an NCE. Even if a compound is considered to be a NCE and the MA applicant is able to gain the prescribed period of data exclusivity, another company nevertheless could also market another version of the medicinal product if such company can complete a full MAA with their own complete database of pharmaceutical tests, preclinical studies and clinical trials and obtain MA for its product.

On April 26, 2023, the EC submitted a proposal for the reform of the European pharmaceutical legislation. The current draft envisages, e.g., a shortening of the periods of data exclusivity, however, there is currently neither a final version of this draft nor a date for its entry into force. While the European Parliament adopted its approving position on the reform on April 10, 2024, no further required legislative steps have been taken since.

### ***Orphan Designation and Exclusivity***

The criteria for designating an orphan medicinal product in the EU are similar in principle to those in the U.S. Under Article 3 of Regulation (EC) 141/2000, a medicinal product may be designated as an orphan product if its sponsor can establish that (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or

chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the EU to justify the necessary investment in its development; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or if such a method exists, the product will be of significant benefit to those affected by the condition, as defined in Regulation (EC) 847/2000. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. An application for orphan drug designation (which is not a marketing authorization, as not all orphan-designated medicines reach the authorization application stage) must be submitted first before an MAA of the medicinal product is submitted. The applicant will receive a fee reduction for the MAA if the orphan drug designation has been granted, but not if the designation is still pending at the time the MAA is submitted, and sponsors must submit annual reports to EMA summarizing the status of development of the medicine. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Designated orphan medicines are eligible for conditional marketing authorization.

The EMA's Committee for Orphan Medicinal Products reassesses the orphan drug designation of a product in parallel with the review for a marketing authorization; for a product to benefit from market exclusivity it must maintain its orphan drug designation at the time of marketing authorization review by the EMA and approval by the EC. Additionally, any marketing authorization granted for an orphan medicinal product must only cover the therapeutic indication(s) that are covered by the orphan drug designation. We have received orphan drug designation from the EMA's Committee for NGN-401 for the treatment of Rett syndrome.

During the 10-year period of market exclusivity, with a limited number of exceptions, the regulatory authorities of the EU member states and the EMA may not accept applications for marketing authorization, accept an application to extend an existing marketing authorization or grant marketing authorization for other similar medicinal products for the same therapeutic indication. A similar medicinal product is defined as a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. An orphan medicinal product can also obtain an additional two years of market exclusivity for an orphan-designated condition when the results of specific studies are reflected in the Summary of Product Characteristics ("SmPC") addressing the pediatric population and completed in accordance with a fully compliant Pediatric Investigation Plan ("PIP"). No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications.

The 10-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, i.e., the condition prevalence or financial returns criteria under Article 3 of Regulation (EC) No. 141/2000 on orphan medicinal products. When the period of orphan market exclusivity for an indication ends, the orphan drug designation for that indication expires as well. Orphan exclusivity runs in parallel with normal rules on data exclusivity and market protection. During the period of market exclusivity, an MA may only be granted to a "similar medicinal product" for the same therapeutic indication if: (i) a second applicant can establish that its product, although similar to the authorized product, is safer, more effective or otherwise clinically superior; (ii) the MA holder for the authorized product consents to a second orphan medicinal product application; or (iii) the MA holder for the authorized product cannot supply enough orphan medicinal product.

In the UK, following the post-Brexit transition period, a system for incentivizing the development of orphan medicines was introduced. Overall, the requirements for orphan designation largely replicate the requirements in the EU and the benefit of market exclusivity has been retained. Products with an orphan designation in the EU can be considered for an orphan marketing authorization in Great Britain, but a UK-wide orphan marketing authorization can only be considered in the absence of an active EU orphan designation. The MHRA will review applications for orphan designation at the time of a marketing authorization, and will offer incentives, such as market exclusivity and full or partial refunds for marketing authorization fees to encourage the development of medicines in rare diseases.

### ***Pediatric Development***

In the EU, companies developing a new medicinal product are obligated to study their product in children and must therefore submit a PIP together with a request for agreement to the EMA, unless the EMA has granted a product-specific waiver, a class waiver, or a deferral for one or more of the measures included in the PIP. The EMA issues a decision on the PIP based on an opinion of the EMA's Pediatric Committee. Companies must conduct pediatric clinical trials in accordance with the PIP approved by the EMA, unless a deferral (e.g., until enough information to demonstrate its effectiveness and safety in adults is available) or waiver (e.g., because the relevant disease or condition occurs only in adults) has been granted by the EMA. The MAA for the medicinal product must include the results of all pediatric clinical trials performed and details of all information collected in compliance with the approved PIP, unless such a waiver or a deferral has been granted. Medicinal products that are granted an MA on the basis of the pediatric clinical trials conducted in accordance with the approved PIP are eligible for a six month extension of the protection under a supplementary protection certificate ("SPC"), provided an application for such extension is made at the same time as filing the SPC application for the product, or at any point up to two years before the SPC expires, or, in the case of orphan medicinal products, a two-year extension of the orphan market exclusivity. This pediatric reward is subject to specific conditions and is not automatically available when data in compliance with the approved PIP are developed and submitted. An approved PIP is also required when an MA holder wants to add a new indication, medicinal form or route of administration for a medicine that is already authorized and covered by intellectual property rights.

In the UK, the MHRA has published guidance on the procedures for UK PIPs which, where possible, mirror the submission format and requirements of the EU system. EU PIPs remain applicable for Northern Ireland and EU PIPs agreed by the EMA prior to January 1, 2021 have been adopted as UK PIPs.

### ***PRIME Designation***

In March 2016, the EMA launched an initiative to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The Priority Medicines ("PRIME") scheme is intended to encourage drug development in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation reviewed under the centralized procedure. Products from small- and medium-sized enterprises may qualify for earlier entry into the PRIME scheme than larger companies on the basis of compelling non-clinical data and tolerability data from initial clinical trials. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and potentially accelerated marketing authorization application assessment once a dossier has been submitted. Importantly, once a candidate medicine has been selected for the PRIME scheme, a dedicated contact point and rapporteur from the CHMP or from EMA's Committee for Advanced Therapies ("CAT") are appointed facilitating increased understanding of the product at EMA's committee level. A kick-off meeting with the CHMP/CAT rapporteur initiates these relationships and includes a team of multidisciplinary experts to provide guidance on the overall development plan and regulatory strategy. PRIME eligibility does not change the standards for product approval, and there is no assurance that any such designation or eligibility will result in expedited review or approval.

### ***Post-Approval Regulation***

Similar to the U.S., both MA holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA, the EC and/or the competent regulatory authorities of the EU member states. This oversight applies both before and after grant of manufacturing licenses and marketing authorizations. It includes control of compliance with EU good manufacturing practices rules, manufacturing authorizations, pharmacovigilance rules and requirements governing advertising, promotion, sale, and distribution, recordkeeping, importing and exporting of medicinal products.

Failure by us or by any of our third-party partners, including suppliers, manufacturers and distributors to comply with EU laws and the related national laws of individual EU member states governing the conduct of clinical trials, manufacturing approval, MA of medicinal products and marketing of such products, both before and after

grant of MA, statutory health insurance, bribery and anti-corruption or other applicable regulatory requirements may result in administrative, civil or criminal penalties. These penalties could include delays or refusal to authorize the conduct of clinical trials or to grant an MA, product withdrawals and recalls, product seizures, suspension, withdrawal or variation of the MA, total or partial suspension of production, distribution, manufacturing or clinical trials, operating restrictions, injunctions, suspension of licenses, fines and criminal penalties.

The holder of an MA for a medicinal product must also comply with EU pharmacovigilance legislation and its related regulations and guidelines, which entail many requirements for conducting pharmacovigilance, or the assessment and monitoring of the safety of medicinal products.

MA holders must establish and maintain a pharmacovigilance system and appoint an individual qualified person for pharmacovigilance, who is responsible for oversight of that system. Key obligations include expedited reporting of suspected serious adverse reactions and submission of Periodic Safety Update Reports (“PSURs”) in relation to medicinal products for which they hold MAs. The EMA reviews PSURs for medicinal products authorized through the centralized procedure. If the EMA has concerns that the risk benefit profile of a product has varied, it can adopt an opinion advising that the existing MA for the product be suspended, withdrawn or varied. The EMA can advise that the MA holder be obliged to conduct post-authorization Phase 4 safety studies. If the EC agrees with the opinion, it can adopt a decision varying the existing MA. Failure by the MA holder to fulfill the obligations for which the EC’s decision provides can undermine the ongoing validity of the MA.

More generally, non-compliance with pharmacovigilance obligations can lead to the variation, suspension or withdrawal of the MA for the product or imposition of financial penalties or other enforcement measures.

The manufacturing process for pharmaceutical products in the EU is highly regulated and regulators may shut down manufacturing facilities that they believe do not comply with regulations. Manufacturing requires a manufacturing authorization, and the manufacturing authorization holder must comply with various requirements set out in the applicable EU laws, regulations and guidance, including Directive 2001/83/EC, Directive 2003/94/EC (repealed by Directive 2017/1572 on January 31, 2022), Regulation (EC) No 726/2004 and the European Commission Guidelines for GMP. These requirements include compliance with cGMP standards when manufacturing pharmaceutical products and active pharmaceutical ingredients, including the manufacture of active pharmaceutical ingredients outside of the EU with the intention to import the active pharmaceutical ingredients into the EU. Amendments or replacements of at least Directive 2001/83/EC and Regulation (EC) No 726/2004 are a part of the reform proposal for European pharmaceutical legislation. Similarly, the distribution of pharmaceutical products into and within the EU is subject to compliance with the applicable EU laws, regulations and guidelines, including the requirement to hold appropriate authorizations for distribution granted by the competent authorities of the EU member states. The manufacturer or importer must have a qualified person who is responsible for certifying that each batch of product has been manufactured in accordance with cGMP, before releasing the product for commercial distribution in the EU or for use in a clinical trial. Manufacturing facilities are subject to periodic inspections by the competent authorities for compliance with cGMP.

### ***Sales and Marketing Regulations***

The advertising and promotion of our product candidates and any products we may develop are also subject to EU laws concerning promotion of medicinal products, interactions with physicians, misleading and comparative advertising and unfair commercial practices. In addition, other national legislation of individual EU member states may apply to the advertising and promotion of medicinal products and may differ from one country to another. These laws require that promotional materials and advertising in relation to medicinal products comply with the product’s SmPC as approved by the national competent authorities. The SmPC is the document that provides information to physicians concerning the safe and effective use of the medicinal product. It forms an intrinsic and integral part of the marketing authorization granted for the medicinal product. Promotion of a medicinal product that does not comply with the SmPC is considered to constitute off-label promotion, which is prohibited in the EU. Direct-to-consumer advertising of prescription-only medicines is also prohibited in the EU. Violations of the rules governing the promotion of medicinal products in the EU could be penalized by administrative measures, fines and imprisonment.

EU regulation with regards to dispensing, sale and purchase of medicines has generally been preserved in the UK following Brexit, through the HMRS. However, organizations wishing to sell medicines online need to register with the MHRA. Following Brexit, the requirements to display the common logo no longer apply to UK-based online sellers, except for those established in Northern Ireland.

### ***Anti-Corruption Legislation***

In the EU, interactions between pharmaceutical companies and physicians are also governed by strict laws, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct both at EU level and in the individual EU member states. The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the EU. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of the EU member states. Violation of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain EU member states also must be publicly disclosed. Moreover, agreements with physicians must often be the subject of prior notification and approval by the physician's employer, his/her regulatory professional organization, and/or the competent authorities of the individual EU member states. These requirements are provided in the national laws, industry codes, or professional codes of conduct, applicable in the individual EU member states. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

### ***Regulation in the UK and Other Markets***

The UK formally left the EU on January 31, 2020, and EU laws now only apply to the UK in respect to Northern Ireland as laid out in the Protocol on Ireland and Northern Ireland and as amended by the Windsor Framework agreed by the UK and EU on February 27, 2023. Amongst other things, the Windsor Framework sets out a long-term set of arrangements for the supply of medicines into Northern Ireland. From January 1, 2025, medicines will need to be approved and licensed on a UK-wide basis by the MHRA, with medicines using the same packaging and labelling across the UK. The EMA will have no role in approving or licensing new drugs for provision in Northern Ireland. The EU and the UK have agreed on a trade and cooperation agreement, which includes specific provisions concerning pharmaceuticals, which include the mutual recognition of cGMP, inspections of manufacturing facilities for medicinal products and cGMP documents issued, but does not provide for wholesale mutual recognition of UK and EU pharmaceutical regulations. At present, Great Britain has implemented EU legislation on the marketing, promotion and sale of medicinal products through the HMRS.

The UK government has adopted the Medicines and Medical Devices Act 2021 ("MMDA") to enable the UK's regulatory frameworks to be updated following the UK's departure from the EU. The MMDA introduces regulation-making, delegated powers covering the fields of human medicines, clinical trials of human medicines, veterinary medicines and medical devices. The MHRA has since been consulting on future regulations for medicines and medical devices in the UK. Specified provisions of the MMDA entered into force on February 11, 2021. The remaining provisions came into effect within two months of February 11, 2021 or will otherwise come into effect as stipulated in subsequent statutory instruments.

For other countries outside of the EU, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials must be conducted in accordance with cGCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension of clinical trials, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

### **Properties and Facilities**

We currently lease two properties for our principal offices: an approximately 42,000 square foot manufacturing facility in Houston, Texas, and an approximately 6,000 square foot office space in New York, New York. The



Houston manufacturing facility is used for our in-house manufacturing, warehouse and cold storage functions, and the lease expires on August 31, 2029. The New York office space is our corporate headquarters, and the lease expires on June 30, 2026. In addition, we have established a hybrid work-from-home policy for many of our employees. We believe these spaces to be sufficient to meet our needs for the foreseeable future and that any additional space we may require will be available on commercially reasonable terms.

In addition to our principal offices, we also lease a combined 39,572 square feet of office and laboratory space in Seattle, Washington that was previously used by Neoleukin prior to the merger for laboratory, discovery, research and development and general and administrative purposes. We have entered into a sublease with an unrelated third party for 6,272 square feet of that space, which will terminate concurrently with the end of our lease for such space on September 30, 2026. We are actively looking for a subtenant for the remaining 33,300 square feet of combined laboratory and office space, which is subject to a lease that will terminate on June 30, 2029. Any net proceeds we receive from any sublease of these properties will be payable to the holders of CVRs issued in connection with the merger, after adjustment for certain costs.

#### **Legal Proceedings**

We may from time to time be named as a party to legal claims, actions and complaints, including matters involving employment, intellectual property or others. We are not presently a party to any legal proceedings that, in the opinion of our management, would reasonably be expected to have a material adverse effect on our business, financial condition, operating results or cash flows if determined adversely to us. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources, and other factors.

## MANAGEMENT

### Directors

Our Board currently consists of six directors and is divided into three classes, with members of each class holding office for staggered three-year terms. Class I directors have terms that expire at the 2027 Annual Meeting of Stockholders; Class II directors have terms that expire at the 2025 Annual Meeting of Stockholders; and Class III directors have terms that expire at the 2026 Annual Meeting of Stockholders.

Our current directors, and their ages, occupations and length of board service as of November 15, 2024, are provided in the table below. Additional biographical descriptions of each director are set forth in the text below the table. These descriptions include the primary individual experience, qualifications, qualities and skills of each of our directors.

Name	Class	Age	Position
Rachel McMinn, Ph.D.	I	51	Executive Chair and Chief Executive Officer
Robert Baffi, Ph.D. <sup>(2)(3)</sup>	III	69	Director
Cory Freedland, Ph.D. <sup>(1)</sup>	I	48	Director
Sarah B. Noonberg, M.D., Ph.D. <sup>(2)</sup>	II	57	Director
Rohan Palekar <sup>(1)(2)</sup>	III	59	Director
Robert Keith Woods (“Keith Woods”) <sup>(1)(3)</sup>	II	57	Director

(1) Member of the Audit Committee

(2) Member of the Compensation Committee

(3) Member of the Nominating Committee

**Rachel McMinn, Ph.D.** Dr. McMinn has served as Chief Executive Officer and Executive Chair of our Board since December 2023, and as founder, Chief Executive Officer and a member of the board of directors of Neurogene OpCo since January 2018. Prior to founding Neurogene, she served as Chief Business and Strategy Officer of Intercept Pharmaceuticals, Inc. (formerly Nasdaq: ICPT, until its acquisition by Alfasigma S.p.A.), a biopharmaceutical company dedicated to the treatment of patients with serious liver disease, from April 2014 to December 2017. Prior to her operational experience, Dr. McMinn was an award-winning biotechnology analyst, with 13 years of experience at firms such as Bank of America Merrill Lynch, Cowen and Company and Piper Jaffray. Dr. McMinn has served on the board of directors of Everyone Medicines since 2021, and prior to that the non-profit Everyone Foundation from 2019 to 2021. Dr. McMinn received her B.A., magna cum laude, from Cornell University and her Ph.D. from The Scripps Research Institute, and was awarded a Post-Doctoral Miller Fellowship at the University of California, Berkeley.

We believe Dr. McMinn is qualified to serve on our Board because of her in-depth knowledge of the Company, her operational and senior management experience, and her extensive healthcare investment research in the biotechnology industry.

**Robert Baffi, Ph.D.** Dr. Baffi has served as a member of our Board since December 2023 and served as a member of the board of directors of Neurogene OpCo from September 2020 to December 2023. Dr. Baffi is a Venture Partner at Samsara BioCapital (“Samsara”), an investment company focused on the life sciences industry, which he joined in March 2021. Dr. Baffi had a 23-year tenure at BioMarin Pharmaceutical Inc. (Nasdaq: BMRN), a global biotechnology company, from May 2000 to March 2023, where he served as President of Global Manufacturing & Technical Operations from 2018 to 2020, and was responsible for overseeing manufacturing, process development based on the baculovirus system, quality, logistics, engineering and analytical chemistry, and led the building of one of the first gene therapy manufacturing facilities of its kind, before he became Senior Advisor to the Chairman and Chief Executive Officer in 2021. Prior to BioMarin, Dr. Baffi served 14 years in a number of increasingly senior positions at Genentech, Inc., primarily in the functional area of quality control. Prior to Genentech, Dr. Baffi worked at Cooper BioMedical, Inc. as a Research Scientist and at the Becton Dickinson Research Center as a Post-Doctoral Fellow. Dr. Baffi has contributed to the approval and commercial success of 28 products. Dr. Baffi has served as a member of the board of directors of Mosaic ImmunoEngineering Inc.

(OTCMTKS: CPMV), a biotechnology company, since June 2021 and Bionic Sight, Inc., a biotechnology company, since 2020. Dr. Baffi also serves on the science advisory board of the National Institute for Bioprocessing Research & Training. Dr. Baffi received his Ph.D., M. Phil. and B.S. in biochemistry from the City University of New York and his M.B.A. from Regis University.

We believe Dr. Baffi is qualified to serve on our Board because of his extensive education and investment, management, commercialization, operational and leadership experience in the life sciences sector.

**Cory Freedland, Ph.D.** Dr. Freedland has served as a member of our Board since December 2023 and served as a member of the board of directors of Neurogene OpCo from February 2019 to December 2023. Dr. Freedland is a Partner at Samsara, an investment company focused on the life sciences industry, which he joined in October 2017. Dr. Freedland has over 20 years of experience, leading multiple successful life science investments in his role. Prior to Samsara, Dr. Freedland was a Principal at Sofinnova Ventures, a biopharmaceutical venture capital firm, where he focused on biopharmaceutical investments. He played a central role in Sofinnova's investments in Civitas Therapeutics, Inc. (acquired by Acorda Therapeutics, Inc.), Principia Biopharma, Spark Therapeutics, Inc. (acquired by Roche), Ziarco Pharma Ltd. (acquired by Novartis AG), and ZS Pharma, Inc. (acquired by AstraZeneca plc). Prior to Sofinnova, Dr. Freedland was a Principal at Novo A/S. Before his transition to healthcare investing, Dr. Freedland was a Vice President in the healthcare investment banking practice at Morgan Stanley. Prior to transitioning to life sciences finance, Dr. Freedland worked as a research scientist for Roche focusing on preclinical drug discovery and novel target identification for psychiatric and neurodegenerative diseases. Dr. Freedland served on the board of directors of Jiya Acquisition Corp. from November 2020 to November 2022. Dr. Freedland has also served on the board of directors of multiple private companies. Dr. Freedland received his Ph.D. in Pharmacology from Wake Forest University School of Medicine, his M.B.A. from the Kellogg School of Management and his B.A. in Psychology and Religious Studies from Connecticut College.

We believe Dr. Freedland is qualified to serve on our Board because of his extensive leadership, investment and business development experience in the life sciences sector, as well as his experience as a director of several biotechnology company boards.

**Sarah B. Noonberg, M.D., Ph.D.** Dr. Noonberg has served as a member of our Board since August 2019. Dr. Noonberg has over 20 years of industry experience leading development programs from discovery to commercialization across a range of indications, and has served as the Chief Medical Officer of Metagenomi, Inc. (Nasdaq: MGX), a next generation gene editing biotechnology company, since January 2023. Prior to Metagenomi, from September 2020 to September 2022, Dr. Noonberg served as the Chief Medical Officer of Maze Therapeutics, a human-genetics driven research and development company, and from May 2018 to May 2019, she served as the Chief Medical Officer of Nohla Therapeutics Inc., a developer of universal, off-the-shelf cell therapies for patients with hematological malignancies and other critical diseases. Prior to Nohla Therapeutics, Dr. Noonberg served as the Chief Medical Officer of Prothena Corporation plc (Nasdaq: PRTA), a biotechnology company, from May 2017 to May 2018. Dr. Noonberg previously served as Group Vice President and Head of Global Clinical Development at BioMarin Pharmaceuticals Inc. (Nasdaq: BMRN), a biotechnology company, from August 2015 to March 2017. From May 2007 to August 2015, she held several positions at Medivation, Inc., a biopharmaceutical company, including as Senior Vice President of Early Development. Dr. Noonberg also previously served as a member of the board of directors of Marinus Pharmaceuticals, Inc. (Nasdaq: MRNS), a biopharmaceutical company, from May 2023 to November 2024, and on the board of directors of Protagonist Therapeutics, Inc (Nasdaq: PTGX), a biopharmaceutical company, from December 2017 to May 2023. Dr. Noonberg received her M.D. from the University of California, San Francisco, her Ph.D. in Bioengineering from the University of California, Berkeley, and her B.S. in Engineering from Dartmouth College. She is a board-certified internist and completed her residency at Johns Hopkins Hospital.

We believe Dr. Noonberg is qualified to serve on our Board because of her senior leadership and public company board experience in the biopharmaceutical industry as well as her extensive medical knowledge and clinical development and regulatory experience.

**Rohan Palekar.** Rohan Palekar has served as a member of our Board since March 2022. Mr. Palekar has served as Chief Executive Officer and a member of the board of directors of 89bio, Inc. (Nasdaq: ETNB), a

biopharmaceutical company, since June 2018. Prior to 89bio, Mr. Palekar held various positions at Avanir Pharmaceuticals, Inc., a specialty pharmaceutical company, including the role of President and Chief Executive Officer of Avanir from December 2015 to July 2017, where he led the company following its acquisition by Otsuka Pharmaceutical Co., Ltd. in 2015. Prior to the acquisition, Mr. Palekar served as Executive Vice President and Chief Operating Officer in 2015 and as Senior Vice President and Chief Commercial Officer of Avanir from March 2012 to March 2015. Prior to Avanir, from 2008 to 2011, Mr. Palekar served as Chief Commercial Officer for Medivation, Inc., a biopharmaceutical company, where he was responsible for all commercial activities, chemistry, manufacturing and controls, medical affairs, and public relations functions. Mr. Palekar also spent over 16 years at Johnson & Johnson (NYSE: JNJ), a diversified healthcare company, in various senior commercial and strategic management roles. Mr. Palekar served as a trustee for Aim High for High School, a non-profit educational institution, from 2018 till 2023 and chair of the board of trustees from 2021 to 2023. Mr. Palekar earned his M.B.A. from the Tuck School of Business at Dartmouth College, his B.Com. in Accounting from the University of Mumbai and his L.L.B. from the University of Mumbai. Mr. Palekar is also a certified Chartered Accountant and a Cost and Management Accountant.

We believe Mr. Palekar is qualified to serve on our Board because of his operational experience in the biopharmaceutical industry as well as his senior management and leadership experience.

**Keith Woods.** Mr. Woods has served as a member of our Board since December 2023. Mr. Woods is an advisor to the board of directors of argenx SE (Nasdaq: ARGX), a biopharmaceutical company, where he served as Chief Operating Officer from April 2018 to March 2023. Mr. Woods has over 30 years of experience in the biopharmaceutical industry. Prior to argenx, Mr. Woods served as Senior Vice President of North American Operations for Alexion Pharmaceuticals, Inc., a biopharmaceutical company, where he managed a team of several hundred people in the U.S. and Canada and was responsible for more than \$1 billion in annual sales. Within Alexion, Mr. Woods had previously served as Vice President and Managing Director of Alexion UK, overseeing all aspects of Alexion's UK business, and Vice President of U.S. Operations and Executive Director of Sales, leading the launch of Soliris in atypical hemolytic uremic syndrome. Prior to Alexion, Mr. Woods held various positions of increasing responsibility within Roche, Amgen Inc. and Eisai Co., Ltd., over a span of 20 years. Mr. Woods has served on the board of directors of X4 Pharmaceuticals, Inc. (Nasdaq: XFOR) since October 2023, Rocket Pharmaceuticals, Inc. (Nasdaq: RCKT) since December 2023, and TScan Therapeutics, Inc. (Nasdaq: TCRX) since December 2023. Mr. Woods received his B.S. in Marketing from Florida State University.

We believe Mr. Woods is qualified to serve on our Board because of his extensive senior management, leadership and operational experience in the biopharmaceutical industry.

#### **Director Independence**

Nasdaq listing rules require a majority of a listed company's board of directors to be comprised of independent directors who, in the opinion of the board of directors, do not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. Subject to specified exceptions, each member of a listed company's audit, compensation and nominating committees must be independent, and audit and compensation committee members must satisfy additional independence criteria under the Exchange Act.

Our Board undertook a review of its composition and the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including the beneficial ownership of our capital stock by each non-employee director, our Board has determined that Drs. Baffi, Freedland and Noonberg and Messrs. Palekar and Woods qualify as "independent directors" as defined by the Nasdaq listing rules. Dr. McMinn is not an independent director because she is our Chief Executive Officer ("CEO"). Former directors Martin Babler, M. Cantey Boyd, Erin Lavelle and Todd Simpson were previously determined to be "independent" under the Nasdaq listing rules.

Our Board also determined that each of the directors currently serving on the Audit Committee and the Compensation Committee satisfy the additional independence criteria applicable to directors on such committees under Nasdaq listing rules and the rules and regulations established by the SEC.

## Executive Officers

Biographical and other information as of November 15, 2024 regarding our executive officers, who are appointed by the Board and serve at the Board's discretion, is set forth below.

Name	Age	Position
Rachel McMinn, Ph.D. <sup>(1)</sup>	51	Executive Chair and Chief Executive Officer
Christine Mikail Cvijic, J.D. ("Christine Mikail")	47	President and Chief Financial Officer
Julie Jordan, M.D.	53	Chief Medical Officer
Stuart Cobb, Ph.D.	54	Chief Scientific Officer

(1) For Dr. McMinn's biographical information, see "Directors" above.

**Christine Mikail, J.D.** Ms. Mikail has served as our President and Chief Financial Officer since December 2023, and as President and Chief Financial Officer of Neurogene OpCo since September 2019. In her role, Ms. Mikail leads Corporate and Program Strategy and Business Development, Portfolio Management, Operations, and Finance. Ms. Mikail brings over two decades of experience supporting biotechnology and pharmaceutical companies in corporate strategy and business development, operations, legal and finance capacities. Prior to Neurogene, Ms. Mikail was Chief Administrative Officer and Head of External Business Development/Alliance Management and General Counsel at Axovant Sciences (which became Sio Gene Therapies Inc. (OTCMAKTS: SIOX)) from March 2015 to March 2017, where she was an integral member of the team that raised \$362 million in the company's initial public offering. Prior to Axovant, she held a variety of senior executive positions at NPS Pharmaceuticals, Inc., Dendreon Corporation, Eli Lilly and Company, and ImClone Systems. Ms. Mikail developed her life sciences focus as a corporate and securities lawyer at international law firms of Reed Smith LLP and Wilmer Cutler Pickering Hale and Dorr LLP. Ms. Mikail received her B.A., cum laude, from Rutgers University and her J.D. from Fordham University School of Law in New York.

**Julie Jordan, M.D.** Dr. Jordan has served as our Chief Medical Officer since January 2024. Prior to Neurogene, Dr. Jordan served as Chief Medical Officer of Homology Medicines, Inc. (formerly Nasdaq: FIXX) from March 2023 to January 2024, where she was responsible for leading clinical development and operations, leading regulatory interactions and supporting translational research programs for the company's pipeline of gene therapy and gene editing candidates for rare diseases, and prior to that, as the company's Senior Vice President, Head of Clinical Development and Operations from February 2022 to March 2023 and Vice President, Clinical Development from May 2021 to February 2022. Prior to Homology, Dr. Jordan served as Senior Director, Global Clinical Development at Cerevel Therapeutics, LLC, a pharmaceutical company, from August 2019 to April 2021, where she was responsible for leading clinical development and operations. Prior to Cerevel, she served as Executive Director, Global Clinical Development at Avanir Pharmaceuticals, Inc., a pharmaceutical company, from March 2019 to July 2019, and Senior Director, Global Clinical Development at Avanir from April 2017 to February 2019, where she was also responsible for leading clinical development and operations. Prior to joining Avanir, Dr. Jordan was a Clinical Instructor of Medicine at Harvard Medical School, treating patients at Massachusetts General Hospital ("MGH"). Dr. Jordan holds an A.B. in Biology from Harvard College and an M.D. from Harvard Medical School and completed her residency in internal medicine at MGH, Harvard Medical School.

**Stuart Cobb, Ph.D.** Dr. Cobb has served as our Chief Scientific Officer since December 2023, and as Chief Scientific Officer of Neurogene OpCo since January 2019. Dr. Cobb brings more than 20 years of experience in translational neuroscience. His expertise is focused on developing genetic therapies for severe neurological and neurodevelopmental disorders. Dr. Cobb leads Neurogene's scientific research, the development of scientific strategy to support Neurogene's existing and growing gene therapy portfolio, and efforts to identify novel technologies that complement Neurogene's pipeline. In addition to his role at Neurogene, Dr. Cobb has been a director of Stuart Cobb Consulting LTD, a scientific consultancy firm since December 2018 and has led a genetic therapy research laboratory as principal investigator within the Medical School at the University of Edinburgh since November 2017 where he currently serves as the Chair of Translational Neuroscience. Prior to these roles, Dr. Cobb was an independent principal investigator and laboratory head from October 1999 to October 2017 and was previously head of the Centre for Neuroscience at the University of Glasgow. He also previously worked at Inveresk

Research International, a contract research organization, from June 1987 to September 1989. Dr. Cobb received his B.Sc. in Pharmacology from the University of Glasgow and Ph.D. (D.Phil.) in Neuroscience from the University of Oxford.

**Family Relationships**

There are no family relationships among any of our directors or executive officers.

## EXECUTIVE COMPENSATION

### Overview

Our named executive officers (“NEOs”) for the year ended December 31, 2023, which consist of each person who served as our principal executive officer and the next two most highly-compensated executive officers who served during the year ended December 31, 2023, prior to and after the Reverse Merger, are:

- After the Reverse Merger:
- Rachel McMinn, Ph.D., our Chief Executive Officer and Executive Chair;
- Christine Mikail, our President and Chief Financial Officer;
- Stuart Cobb, Ph.D., our Chief Scientific Officer;
- Prior to the Reverse Merger:
- Donna Cochener, our former Interim Chief Executive Officer and General Counsel;
- Jonathan Drachman, M.D., our former Chief Executive Officer and President; and
- Sean Smith, our former Interim Chief Financial Officer

### 2023 Summary Compensation Table

The following table summarizes the compensation awarded to, earned by, or paid to our NEOs during the years ended December 31, 2023 and 2022.

Name and Principal Position	Year	Salary (\$)	Bonus (\$) <sup>(1)</sup>	Option Awards (\$) <sup>(2)</sup>	Non-Equity Incentive Plan Compensation (\$) <sup>(3)</sup>	All Other Compensation (\$) <sup>(4)</sup>	Total (\$)
<b>NEOs After the Reverse Merger</b>							
Rachel McMinn	2023	467,890	196,514	404,087	—	12,138	1,080,629
Founder and Chief Executive Officer	2022	447,741	205,961	—	—	2,843	656,545
Christine Mikail	2023	455,787	167,502	509,195	—	—	1,132,484
President and Chief Financial Officer	2022	436,160	175,554	—	—	—	611,714
Stuart Cobb <sup>(5)</sup>	2023	362,258	116,549	305,517	—	—	784,324
Chief Scientific Officer	2022	341,256	117,731	—	—	—	458,987
<b>NEOs Prior to the Reverse Merger</b>							
Donna Cochener	2023	423,581	219,375	—	264,935	919,541	1,827,432
Former Interim Chief Executive Officer and General Counsel	2022	290,986	—	590,146	108,247	4,520	993,899
Jonathan Drachman	2023	113,544	—	—	—	436,439	549,983
Former Chief Executive Officer and President	2022	452,985	—	251,655	192,519	5,020	902,179
Sean Smith Former Interim Chief Financial Officer	2023	384,102	159,167	—	192,223	1,074,291	1,809,783

(1) For individuals who were NEOs after the Reverse Merger, amounts in this column represent annual discretionary bonuses for services performed during the fiscal year that were paid early in the following fiscal year. Amounts for Ms. Cochener and Mr. Smith for 2023 represent a retention bonus of \$219,375 for Ms. Cochener and \$159,167 for Mr. Smith paid in connection with their separation from the Company effective upon the closing of the Reverse Merger.

(2) Amounts in this column represent the aggregate grant date fair value, computed in accordance with Financial Accounting Standards Board (“FASB”) Accounting Standards Codification (“ASC”) Topic 718, of stock options granted to the NEOs in the years shown. For more information regarding the assumptions used in these calculations, see Note 14 to our consolidated financial statements, Stock-Based

Compensation, in our Annual Report on Form 10-K for the year ended December 31, 2023. These amounts may not correspond to the actual value eventually realized by each NEO because the value realized depends on the market value of our common stock at the time the stock option is exercised.

- (3) Amounts for Dr. Drachman in this column as well as the 2022 amount for Ms. Cochener in this column represent compensation earned under Neoleukin's 2022 incentive compensation plan, which provided for an annual incentive compensation payment, subject to achievement of Neoleukin's performance goals and individual achievement. Amounts for Ms. Cochener and Mr. Smith in this column for 2023 relate to compensation earned pursuant to the amendments to their Executive Employment Agreements which provided for payment of a minimum of 100% of the target annual bonus set forth in those amendments, pro-rated to the date of separation from the Company, as increased based on a determination by the Board that each of Ms. Cochener and Mr. Smith had exceeded the corporate goals set for them in 2023.
- (4) Amounts in this column include matching contributions under our 401(k) plan. For NEOs prior to the Reverse Merger, amounts in this column also include a telephone and transportation benefit. Amounts for Ms. Cochener, Dr. Drachman and Mr. Smith for 2023 also include payments in connection with their separation from the Company pursuant to the Separation Agreement and Release that each of them entered into with Neoleukin. Such agreements are described below in the section titled "Potential Payments Upon Termination or Change in Control."
- (5) Amounts included as "Base Salary" for Dr. Cobb were paid as a consulting fee to Stuart Cobb Consulting Ltd. (the "Consultant") and amounts included as "Bonus" for Dr. Cobb were paid as a success fee to the Consultant, in each case pursuant to a consulting agreement between the Company and the Consultant under which Dr. Cobb provides services to the Company as the Chief Scientific Officer. This agreement is described below in the section titled "Employment Agreements and Offer Letters."

#### **Outstanding Equity Awards at 2023 Fiscal-Year End Table**

The following table sets forth information regarding the outstanding equity awards held by each NEO as of December 31, 2023, as adjusted for the 1-for-5 reverse stock split effected September 25, 2023, the 1-for-4 reverse stock split effected December 18, 2023 (the "1-for-4 Reverse Stock Split") and the subsequent Closing on the same date.



Name	Grant Date <sup>(1)</sup>	Option Awards		Option Exercise Price (\$)	Option Expiration Date
		Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable		
<b>NEOs After the Reverse Merger</b>					
Rachel McMinn <sup>(2)</sup>	3/11/2019	7,087	—	6.40	3/10/2024
	10/16/2020	14,690	3,866	11.79	10/15/2025
	9/23/2021	425	331	25.32	9/22/2026
	3/9/2023	—	37,800	20.22	3/8/2028
Christine Mikail	10/1/2019	29,452	—	5.82	9/30/2029
	10/16/2020	29,925	7,875	10.71	10/15/2030
	9/23/2021	425	331	23.02	9/22/2031
	3/9/2023	—	37,800	18.39	3/8/2033
Stuart Cobb	3/11/2019	13,513	—	5.82	3/10/2029
	10/16/2020	17,955	4,725	10.71	10/15/2030
	9/23/2021	8,634	6,717	23.02	9/22/2031
	3/9/2023	—	22,680	18.39	3/8/2033
<b>NEOs Prior to the Reverse Merger</b>					
Donna Cochener	3/14/2022	19,999	—	36.20	3/13/2032
	8/2/2022	4,999	—	19.80	8/1/2032
Jonathan Drachman	8/31/2019	73,906	—	56.00	9/30/2024
	8/10/2020	13,562	—	240.00	9/30/2024
	8/3/2021	7,916	—	136.00	9/30/2024
	8/2/2022	14,582	—	19.80	9/30/2024
Sean Smith	10/10/2019	1,300	—	54.20	10/9/2029
	8/10/2020	750	—	240.00	8/10/2030
	8/3/2021	1,198	—	136.00	8/2/2031
	3/2/2022	3,999	—	54.40	3/1/2032
	8/2/2022	7,499	—	19.80	8/1/2032

- (1) These stock options vest as to 25% of the award on the one-year anniversary of the vesting commencement date, which is the date of grant, and in 36 equal monthly installments thereafter.
- (2) Grants made to Dr. McMinn in 2019-2023 have an exercise price that is 110% of the fair market value of the common stock on the date of grant in compliance with the requirements of Section 422 of the Internal Revenue Code of 1986, as amended, relating to grants of incentive stock options to individuals who hold 10% or more of the outstanding stock of the issuer on the date of grant.

## Employment Agreements and Offer Letters

### *NEOs After the Reverse Merger*

#### *Rachel McMinn*

Dr. McMinn executed an offer letter with Neurogene OpCo on January 10, 2019, which sets forth conditions of Dr. McMinn’s at-will employment as Neurogene OpCo’s President and Chief Executive Officer. Dr. McMinn also executed Neurogene OpCo’s standard form of Proprietary Information and Inventions Assignment Agreement. Dr. McMinn’s offer letter entitled her to an initial base salary at the monthly rate of \$33,333, and she was eligible to receive a target annual bonus of 40% of her annual base salary, payable at the discretion of the Neurogene board of directors. In addition, Dr. McMinn’s offer letter provided that Dr. McMinn will be able to participate in any current and future employee equity programs, and that any stock award will be subject to the terms of Neurogene’s 2018

Equity Incentive Plan (the “2018 Plan”) and a restricted stock agreement to be entered into between Dr. McMinn and Neurogene OpCo. In January 2022, Dr. McMinn’s annual salary was increased to \$467,890.

On April 1, 2024, we entered into an Executive Employment Agreement (the “McMinn Employment Agreement”) with Dr. McMinn, pursuant to which she is entitled to an initial annual base salary of \$595,000, subject to increases from time to time as determined by the Board. She is also eligible under the McMinn Employment Agreement to receive an annual performance bonus targeted at 55% of her annual base salary, or such other amount as determined by the Board or a committee of the Board based on the achievement of any applicable bonus objectives and/or conditions set by the Board or a committee of the Board and subject to her continued employment with the Company through the date of payment of such annual bonus. Dr. McMinn will also be eligible to receive annual equity-based incentive awards as determined by the Board or a committee of the Board. The McMinn Employment Agreement also provides for severance payments and benefits in connection with certain terminations of employment as described in the section “Potential Payments Upon Termination or Change of Control.”

*Christine Mikail*

Ms. Mikail executed an offer letter with Neurogene OpCo effective as of September 3, 2019, which sets forth conditions of Ms. Mikail’s at-will employment as Neurogene OpCo’s President and Chief Financial Officer. Ms. Mikail also executed Neurogene OpCo’s standard form of Proprietary Information and Inventions Assignment Agreement. Ms. Mikail’s offer letter entitled her to an initial annual base salary of \$390,000, and she was eligible to receive a target annual bonus of 35% of her annual base salary, payable at the discretion of the Neurogene board of directors. In addition, pursuant to Ms. Mikail’s offer letter, following the commencement of Ms. Mikail’s employment, Neurogene OpCo recommended to its board of directors that Ms. Mikail be granted an option to purchase shares of Neurogene OpCo common stock (which, after giving effect to the Merger Exchange Ratio, is equal to 41,580 shares of Neurogene common stock), subject to the terms of the 2018 Plan and a stock option agreement entered into between Ms. Mikail and Neurogene OpCo, and such option award was approved by the Neurogene OpCo board of directors on September 5, 2019 and granted on October 1, 2019. Consistent with the terms of the offer letter and stock option agreement, this option vested in full on the fourth anniversary of the vesting commencement date. Ms. Mikail’s offer letter also provides for severance benefits in connection with certain terminations of employment, as described in the section titled “Potential Payments Upon Termination or Change of Control.”

On April 1, 2024, we entered into an Executive Employment Agreement (the “Mikail Employment Agreement”) with Ms. Mikail, pursuant to which she is entitled to an initial annual base salary of \$515,000, subject to increases from time to time as determined by the Board. She is also eligible under the Mikail Employment Agreement to receive an annual performance bonus targeted at 45% of her annual base salary, or such other amount as determined by the Board or a committee of the Board based on the achievement of any applicable bonus objectives and/or conditions set by the Board or a committee of the Board and subject to her continued employment with the Company through the date of payment of such annual bonus. Ms. Mikail will also be eligible to receive annual equity based incentive awards as determined by the Board or a committee of the Board. The Mikail Employment Agreement also provides for severance payments and benefits in connection with certain terminations of employment as described in the section “Potential Payments Upon Termination or Change of Control.”

*Stuart Cobb*

On December 13, 2018, Neurogene OpCo entered into a Consulting Agreement with Stuart Cobb Consulting Ltd., Dr. Cobb’s consulting company (the “Consultant”) pursuant to which Consultant makes available to the Company Dr. Cobb’s services to the Company as its Chief Scientific Officer. This Consulting Agreement was subsequently amended four times, effective January 1, 2020; July 13, 2020; April 1, 2022 and January 1, 2023. We refer to the Consulting Agreement and each of these amendments as the “Amended Consulting Agreement.”

Pursuant to the Amended Consulting Agreement, Dr. Cobb’s the Consultant was entitled to receive a monthly payment of \$30,833 in 2023 for services provided by him to the Company as Chief Scientific Officer. The term of the Amended Consulting Agreement was for five years from the date of the initial contract, with the ability for the parties to extend such term based on their mutual agreement.

On April 19, 2024, we entered into an Amended and Restated Consulting Agreement with the Consultant (the “Restated Consulting Agreement”) pursuant to which Dr. Cobb continues to serve as our Chief Science Officer. The Restated Consulting Agreement provides that the Consultant will receive a minimum annual consulting fee of \$440,000 in exchange for Dr. Cobb’s services as Chief Scientific Officer, to be paid in equal monthly installments. The Restated Consulting Agreement also provides that Consultant is eligible to receive an annual success fee targeted at 40% of the Consultant’s annualized consulting fee determined by the Board or a committee of the Board that Dr. Cobb has achieved the performance parameters established by the Board for such year, subject to Dr. Cobb continued active engagement with the Company through the payment date of such annual success fee. The Restated Consulting Agreement also provides for severance payments and benefits in connection with certain terminations of employment as described in the section “Potential Payments Upon Termination or Change of Control.”

### ***NEOs Prior to the Reverse Merger***

#### *Donna Cochener*

We entered into an employment agreement with Donna Cochener effective as of March 14, 2022, setting forth the terms of Ms. Cochener’s employment as our General Counsel, SVP Legal.

On the effective date of the employment agreement, Ms. Cochener was granted an option to purchase 20,000 shares (after giving effect to the 1-for-5 Reverse Stock Split and the 1-for-4 Reverse Stock Split) of common stock with an exercise price of \$36.20 per share. The option was to vest and become exercisable pursuant to that award agreement with respect to (a) 1/4th of the total underlying shares on the first anniversary of the grant date and (b) with respect to 1/48th of the total underlying shares on a monthly basis thereafter such that the option would have been fully vested and exercisable on the fourth anniversary of the grant date, subject to Ms. Cochener’s continuous service through each applicable vesting date. Pursuant to her initial employment agreement, if Ms. Cochener experienced a termination without “cause” or resigned for “good reason” (each as defined in Ms. Cochener’s employment agreement), Ms. Cochener also have been entitled to receive (i) continued base salary for nine months, payable in accordance with our standard payroll practices, and (ii) premium payments for continued healthcare coverage for up to nine months. If Ms. Cochener experienced a termination without “cause” or she resigned for “good reason” during the 12-month period following a change in control of the Company (as defined in Ms. Cochener’s employment agreement), then in lieu of the foregoing, Ms. Cochener would have been entitled to (A) continued base salary for 12 months, payable in accordance with the Company’s standard payroll practices; (B) 100% of her annual target bonus, payable in a single lump-sum; (C) premium payments for continued healthcare coverage for up to 12 months; and (D) full accelerated vesting of her then-outstanding equity awards.

In connection with Ms. Cochener’s appointment as Interim Chief Executive Officer, we entered into an amendment to her executive employment agreement dated April 3, 2023 and effective March 31, 2023 (the “Cochener Employment Agreement Amendment”). The Cochener Employment Agreement Amendment provided that Ms. Cochener would be paid an initial base salary of \$450,000 for her role as Interim Chief Executive Officer. Ms. Cochener was entitled to (a) an annual bonus for 2023, which replaced the annual bonus described in the employment agreement, of not less than \$219,375, which was to be prorated in the event Ms. Cochener was terminated without “cause” or resigned for “good reason” prior to December 31, 2023 (b) a retention bonus of \$219,375, payable upon (i) a “change of control” (as defined in the Cochener Employment Agreement Amendment), (ii) termination by the Company without “cause” or resignation by Ms. Cochener for “good reason” or (iii) December 31, 2023; provided that Ms. Cochener remained in her role through the triggering event for such payment. In the event Ms. Cochener was terminated without “cause” or resigned for “good reason” outside of a “change of control”, Ms. Cochener would have been entitled to receive a separation payment equivalent to nine months of salary, payable as salary continuation, as well as nine months of COBRA benefits for herself and her family. In the event of a termination without “cause” or resignation with “good reason” within six months prior to or twelve months following a “change of control”, Ms. Cochener was entitled to receive a lump sum payment equal to (a) 15 months of her base salary, (b) 125% of her annual bonus amount, (c) 15 months of COBRA coverage for herself and her family, (d) acceleration of all outstanding equity awards and (e) an extension of the post-separation exercise period of her stock options to 15 months after separation; provided, that in the event the termination or resignation preceded the “change of control”, such “change in control” occurred by March 1 of the following year. All

severance payments were conditioned on receipt of a standard release of claims from Ms. Cochener at the time of separation.

Ms. Cochener's employment was terminated at the Closing and the termination was considered to be without "cause" related to a change in control for purposes of her employment agreement, as amended by the Cochener Employment Agreement Amendment. Ms. Cochener entered into a Separation Agreement and Release with the Company (the "Cochener Separation Agreement") pursuant to which she received certain separation payments and benefits in return for providing a release of claims against the Company as of her last day of employment on December 18, 2023 (the "Separation Date"). Pursuant to the terms of the Cochener Separation Agreement, Ms. Cochener received (a) a lump sum cash severance payment equivalent to 125% months of her annual base salary and target annual bonus on the Separation Date, (b) a lump-sum payment representing fifteen months of health insurance premiums at a rate approved by the Board of Directors of Neoleukin, (c) a lump-sum payment representing an annual bonus of 125% of her target annual bonus for 2023, pro-rated to the Separation Date, (d) accelerated vesting of all outstanding unvested option awards she held as of the Separation Date (as agreed in her employment agreement), and (e) an extension of the post-termination exercise period in which she may exercise the vested and exercisable options pursuant to her outstanding option grants for fifteen months following the termination of her continuous service to the Company. Ms. Cochener entered into a Consulting Agreement with the Company effective as of the time of the Merger Closing, to be in effect until March 31, 2024.

On February 15, 2024, following a period of consultancy, we hired Ms. Cochener as Senior Vice President, General Counsel. Ms. Cochener is not serving as an executive officer of the Company in this role. Her prior consulting agreement with the Company was terminated by mutual agreement as of the date of her re-hire.

#### *Jonathan Drachman*

We entered into an amended and restated executive employment agreement effective April 15, 2020 with our former Chief Executive Officer, Dr. Drachman, to amend the terms of severance payments and benefits to which Dr. Drachman would have been entitled in the event of certain terminations of his employment and to modify certain other terms. The amended employment agreement amended and restated the prior employment agreement between the Company and Dr. Drachman, dated August 5, 2019.

Pursuant to the amended employment agreement, Dr. Drachman's initial base salary was \$425,000, subject to increases from time to time as determined by the Compensation Committee. Dr. Drachman was also eligible to receive an annual bonus of up to a target level of 50% of his base salary, with the actual bonus payment amount to be as determined by the Compensation Committee. Pursuant to that agreement, in the event that Dr. Drachman was terminated without "cause" or for "good reason" (each as defined in the amended employment agreement) unrelated to a change in control of the Company (as defined in Dr. Drachman's amended employment agreement), subject to Dr. Drachman's execution and non-revocation of a release of claims, Dr. Drachman was entitled to receive (a) continued base salary for 12 months, payable in accordance with the Company's standard payroll practices; (b) premium payments for continued healthcare coverage for up to 12 months; and (c) solely in the case of equity awards outstanding as of April 15, 2020, accelerated vesting of the portion of such outstanding equity awards that would have vested and become exercisable, as applicable, if he had remained in service for an additional 12 months following his date of termination. In the event Dr. Drachman experienced a termination without "cause" or he resigned for "good reason" during the 12-month period following a change in control of the Company, then in lieu of the foregoing, Dr. Drachman would have been entitled to (i) continued base salary for 18 months, payable in accordance with the Company's standard payroll practices; (ii) 150% of his annual target bonus, payable in a single lump-sum; (iii) premium payments for continued healthcare coverage for up to 18 months; and (iv) accelerated vesting of his then-outstanding equity awards.

On March 8, 2023, we entered into a Separation Agreement with Dr. Drachman, pursuant to which Dr. Drachman resigned from the Company on March 31, 2023 (the "Drachman Separation Agreement"). Pursuant to the Drachman Separation Agreement, in exchange for providing certain releases for the benefit of the Company, Dr. Drachman was entitled to receive (a) cash severance payments equivalent to his base salary for 12 months following the Drachman Separation Date in the form of salary continuation payments, payable in accordance with the Company's standard payroll practices; (b) premium payments for continued healthcare coverage for up to 12 months

following the Drachman Separation Date; (c) a lump sum cash payment equivalent to three months' worth of Dr. Drachman's annual target bonus for 2023, (d) accelerated vesting of 100% of the option grant awarded to Dr. Drachman on August 2, 2022 (the "Drachman Grant"), such that the Drachman Grant shall have been fully vested and exercisable on the Drachman Separation Date, and (e) extension of the post-termination exercise period in which Dr. Drachman may exercise all vested and exercisable option awards for 18 months following the date of the Drachman Separation Agreement.

*Sean Smith*

We entered into an employment agreement with Sean Smith effective as of August 3, 2022, setting forth the terms of Mr. Smith's employment as our Vice President of Finance and acting Principal Accounting Officer.

Pursuant to the employment agreement, if Mr. Smith experienced a termination without "cause" or resigned for "good reason" (each as defined in Mr. Smith's employment agreement), Mr. Smith would have been entitled to receive (i) continued base salary for nine months, payable in accordance our standard payroll practices, and (ii) premium payments for continued healthcare coverage for up to nine months. In the event Mr. Smith experienced a termination without "cause" or he resigned for "good reason" during the 12-month period following a change in control of the Company, then in lieu of the foregoing, Mr. Smith would have been entitled to (A) continued base salary for 12 months, payable in accordance with the Company's standard payroll practices; (B) 100% of his annual target bonus, payable in a single lump sum, (C) premium payments for continued healthcare coverage for up to 12 months' and (D) full accelerated vesting of his then-outstanding equity awards.

In connection with Mr. Smith's appointment as Interim Chief Financial Officer, we entered into an amendment to his executive employment agreement, dated April 3, 2023 and effective March 31, 2023 (the "Smith Employment Agreement Amendment"). The Smith Employment Agreement Amendment provided that Mr. Smith would be paid an initial base salary of \$410,000 for his role as Interim Chief Financial Officer. Mr. Smith was entitled to (a) an annual bonus for 2023, which replaced the annual bonus described in the previous employment agreement, of not less than \$159,167, which was to be prorated in the event Mr. Smith was terminated without "cause" or resigned for "good reason" prior to December 31, 2023, (b) a retention bonus of \$159,167 payable upon (i) a "change of control" (as defined in the Smith Employment Agreement Amendment), (ii) termination by the Company without "cause" or resignation by Mr. Smith for "good reason" or (iii) December 31, 2023, provided that Mr. Smith had remained in his role through the triggering event for such payment. In the event Mr. Smith was terminated without "cause" or resigned for "good reason" outside of a "change in control", Mr. Smith would have be entitled to receive a separation payment equivalent to nine months of salary, payable as salary continuation, as well as nine months of COBRA benefits for himself and his family. In the event of a termination without "cause" or resignation with "good reason" within six months prior to or twelve months following a "change of control", Mr. Smith would have be entitled to receive a lump sum payment equal to (a) 15 months of his base salary, (b) 125% of his annual bonus amount, (c) premium payments for 15 months of COBRA coverage for himself and his family, (d) acceleration of all outstanding equity awards and (e) an extension of the post-separation exercise period of his stock options to 15 months after separation; provided, that in the event the termination or resignation preceded the "change in control", such "change of control" occurred by March 1 of the following year. All severance payments would be conditioned on receipt of a standard release of claims from Mr. Smith at the time of separation.

Mr. Smith's employment was terminated effective at the Closing and was considered to be without "cause" related to a change in control for purposes of his employment agreement. Mr. Smith entered into a Separation Agreement and Release with the Company (the "Smith Separation Agreement") pursuant to which Mr. Smith received certain separation payments and benefits in return for providing a release of claims against the Company as of his last day of employment on December 18, 2023 (the "Separation Date"). Pursuant to the terms of the Smith Separation Agreement, Mr. Smith received (a) a lump sum cash severance payment equivalent to 125% months of his annual base salary and target annual bonus on the Separation Date, (b) a lump-sum payment representing fifteen months of health insurance premiums at a rate approved by the Board of Directors of Neoleukin, (c) a lump-sum payment representing an annual bonus of 125% of his target annual bonus for 2023, pro-rated to the Separation Date, (d) accelerated vesting of all outstanding unvested option awards held by Mr. Smith as of the Separation Date (as agreed in the Smith Employment Agreement Amendment), (e) accelerated vesting and issuance of the shares underlying an award of restricted stock units granted to Mr. Smith in February 2022, which would have vested on

February 1, 2024 had Mr. Smith continued to be employed by the Company through that date and (f) an extension of the post-termination exercise period in which Mr. Smith may exercise the vested and exercisable options pursuant to his outstanding option grants for fifteen months following the termination of his continuous service to Neoleukin. Mr. Smith entered into a Consulting Agreement with the Company effective as of the time of the Closing, which was in effect until April 1, 2024.

### Base Salaries

Base salaries are intended to provide a level of compensation sufficient to attract and retain an effective management team, when considered in combination with the other components of our executive compensation program. The relative levels of base salary for our NEOs are designed to reflect each NEO's scope of responsibility and accountability. Effective January 1, 2024, Dr. McMinn's annual base salary was increased from \$467,890 to \$595,000, Ms. Mikail's base salary was increased from \$455,787 to \$515,000 and the annual payments to the Consultant for services provided to the Company by Dr. Cobb was increased from \$362,295 to \$440,000. The increase in base salaries and consulting fees reflects in part the transition of Neurogene from a private company to a public company pursuant to the Reverse Merger. For information on the base salaries for our NEOs prior to the Reverse Merger, refer to the "2023 Summary Compensation Table" and the section titled "Employment Agreements and Offer Letters" above.

### Incentive Compensation

#### NEOs After the Reverse Merger

**Annual Cash Bonuses.** Our annual incentive program is intended to reward our NEOs for performance during a fiscal year. From time to time, our compensation committee or the Board, as applicable, in their discretion may approve annual incentives for our NEOs based on individual performance, company performance, or as otherwise determined appropriate. Each of our NEOs was eligible to receive a target bonus at the discretion of the Board with respect to 2023 (as a percentage of base salary) subject to the Company's and/or the NEO's achievement of specific performance goals. The following table sets forth the target annual bonus percentages and the actual bonuses paid for Dr. McMinn and Ms. Mikail and the success fee paid to the Consultant related to Dr. Cobb's services for the fiscal year ended December 31, 2023.

Name	Target Annual Cash Bonus (% of Base Salary) <sup>(1)</sup>	Target Annual Cash Bonus (\$)	Actual Annual Cash Bonus (\$)
Rachel McMinn	40 %	\$ 187,156	\$ 196,514
Christine Mikail	35 %	\$ 159,525	\$ 167,602
Stuart Cobb	30 %	\$ 110,999	\$ 116,549

(1) Target percentages for 2023. Effective January 1, 2024, Dr. McMinn's target bonus is 55%, Ms. Mikail's target bonus amount is 45%, and Dr. Cobb's target bonus is 40%.

**Equity Awards.** We have historically provided long-term incentive compensation to our NEOs through grants of stock options to purchase shares of our common stock under our 2018 Plan. In general, stock options vest as to 25% of the award on the first anniversary of the applicable vesting commencement date and then the remaining options vest in equal monthly installments on the last day of each month over the following 36 months, subject to our NEO's continued employment through the applicable vesting date. During the year ended December 31, 2023, we made grants of stock options to each of Dr. McMinn, Ms. Mikail and Dr. Cobb. The grant date fair values of such awards are set forth in the "2023 Summary Compensation Table" above, and the number of shares underlying such awards and the vesting terms of such awards are set forth in the "2023 Summary Compensation Table" and the "Outstanding Equity Awards at 2023 Fiscal-Year End Table" above.

### *NEOs Prior to the Reverse Merger*

For information on the incentive compensation arrangements for the NEOs prior to the Merger, refer to the “2023 Summary Compensation Table,” the “Outstanding Equity Awards at 2023 Fiscal-Year End Table” and the section titled “Employment Agreements and Offer Letters” above.

### **Retirement Benefits**

We do not maintain, and no NEO is eligible to participate in, any defined benefit pension plan or nonqualified deferred compensation plan. Each of our current NEOs is eligible to participate in a tax-qualified 401(k) savings plan, which allows eligible participants to defer a portion of their compensation, within the limits prescribed by the Internal Revenue Code of 1986, as amended, and the applicable limits under the 401(k) plan, on a pre-tax or after-tax (Roth) basis, through contributions to the 401(k) plan. Pursuant to the terms of such 401(k) plan, we match 100% of the employees contributions up to the first three percent of employee’s eligible compensation and 50% of the employee contributions up to the next two percent of the employee’s eligible compensation.

### **Potential Payments Upon Termination or Change in Control**

#### *Rachel McMinn*

Pursuant to the terms set forth in an amendment to Dr. McMinn’s Restricted Stock Purchase Agreement, in the event that Dr. McMinn is terminated by Neurogene other than for “Cause” or that Dr. McMinn resigns for “Good Reason” within 60 days prior to, or within 12 months following, a “Change in Control,” then the vesting schedule of her stock options will be accelerated so that all unvested stock options will immediately become vested on such date.

For purposes of Dr. McMinn’s vesting acceleration:

- “Cause” generally means Dr. McMinn’s: (i) repeated and willful failure after written notice to perform her reasonably assigned duties for Neurogene, (ii) engagement in dishonesty, gross negligence or misconduct or (iii) conviction of, or the entry of a pleading of guilty or nolo contendere of, any crime involving moral turpitude or any felony.
- “Change in Control” generally means (i) a merger or consolidation in which Neurogene or its subsidiary is a constituent party and Neurogene issues shares of Neurogene capital stock pursuant to such merger or consolidation, or (ii) the sale, lease, transfer, exclusive license or other disposition, in a single transaction or series of related transactions, by Neurogene of all or substantially all the assets of Neurogene.
- “Good Reason” generally means (i) where Dr. McMinn and Neurogene mutually agree in writing that Good Reason exists, (ii) a material diminution in annual base salary (excluding across the board reductions), (iii) any material diminution in title, authority, responsibilities or lines of reporting, or (iv) a required geographic relocation by more than 50 miles, in each case, subject to standard notice and cure periods.

On April 1, 2024, we entered into the McMinn Employment Agreement, pursuant to which Dr. McMinn will entitled to receive the following severance amounts upon termination by the Company for “cause” or by Dr. McMinn for “good reason” (in each case as defined in the McMinn Employment Agreement), subject to Dr. McMinn’s execution and non-revocation of a release of claims in favor of the Company and continued compliance with certain restrictive covenants:

- a lump sum payment equal to 12 months of her annual base salary then in effect;
- any bonus earned for the fiscal year prior to such termination but not yet paid;
- a pro-rata annual bonus amount calculated based on the number of days from and including the first day of the then-current fiscal year to the date of termination; and
- premium payments for up to 12 months of continuing health care coverage benefits for Dr. McMinn and any dependents who are covered by her health care benefits.

If Dr. McMinn is terminated by the Company without “cause” or resigns for “good reason” during a period that is three months prior to or twelve months following a change in control, then in lieu of the foregoing, Dr. McMinn will be entitled to receive:

- a lump sum payment equal to 1.5x the sum of her annual base salary and target annual bonus then in effect;
- any bonus earned for the fiscal year prior to such termination but not yet paid;
- premium payments for up to 18 months of continuing health care coverage benefits for Dr. McMinn and any dependents who are covered by her health care benefits; and
- acceleration of the vesting of all equity and equity-based awards held by Dr. McMinn as of such termination

For purposes of the McMinn Employment Agreement:

- “Cause” generally means a good faith determination by the Board that her employment be terminated due to her indictment, conviction or plea to a felony or other crime involving fraud, dishonesty or moral turpitude, material misconduct or gross negligence in performing her duties, her material failure or refusal to follow policies and lawful directives of the Board, any fraud, embezzlement, theft or dishonesty in connection with her employment by the Company, her material breach of the McMinn Employment Agreement or her failure to comply in any material respect with applicable laws with respect to the operation of the business.
- “Good Reason” generally means the failure or refusal of the Company to comply with the terms of the McMinn Employment Agreement in any material respect, a material diminution in Dr. McMinn’s duties, title, authority, status or responsibilities, a material reduction in her base salary or annual cash bonus opportunity (in each case unless it is part of a reduction that applies similarly and in substantially the same way to similarly situated officers), or requiring Dr. McMinn to be located at an office that is more than 50 miles from the Company’s current headquarters (other than pursuant to a stay home order from a governmental entity).
- “Change in Control” generally means any person becoming the beneficial owner of more than 50% of the securities of the Company (directly or indirectly), a change in the majority of the Board of Directors, consummation of a merger or other acquisition pursuant to which the beneficial owners of the Company prior to such transaction no longer hold a majority of the voting securities of the Company following such transaction, the implementation of a plan of dissolution or liquidation, or the consummation of a sale of more than 50% of the Company’s assets to an entity whose beneficial ownership is less than 50% owned by the prior beneficial owners of the Company in substantially the same proportions as before such transaction.

#### *Christine Mikail*

Pursuant to the terms set forth in Ms. Mikail’s offer letter, in the event that Ms. Mikail is terminated by Neurogene other than for “Cause” or that Ms. Mikail resigns for “Good Reason,” then Ms. Mikail will be entitled to the following severance benefits: (i) a lump sum severance payment equal to six months of Ms. Mikail’s base salary, (ii) any unpaid annual bonus amount earned by Ms. Mikail with respect to the calendar year ended prior to such termination, and (iii) Neurogene-subsidized COBRA continuation premiums for up to six months. If such termination is within 12 months following a Change in Control, then the lump sum severance payment that Ms. Mikail is entitled to will equal 12 months of Ms. Mikail’s base salary and the vesting of her stock options will be accelerated so that all unvested stock options will immediately become vested on the date that is 60 days after such termination. All severance benefits are conditioned on Ms. Mikail’s execution and non-revocation of a release of claims in favor of Neurogene and continued compliance with restricted covenants.



For purposes of Ms. Mikail's offer letter:

- "Cause" generally means Ms. Mikail's: (i) indictment or conviction, or entry of a pleading of guilty or no contest, with respect to a felony or another crime involving fraud, dishonesty or moral turpitude, (ii) material misconduct or gross negligence in the performance of assigned duties to Neurogene, (iii) material failure or refusal to (A) follow policies or lawful directives established by the Neurogene Chief Executive Officer or the Neurogene board of directors or (B) perform duties or obligations, subject to a 30-day cure period, (iv) act of fraud, embezzlement, theft or dishonesty in the course of employment with Neurogene, (v) material breach of Ms. Mikail's offer letter, Neurogene's policies, the Employee Proprietary Information and Inventions Assignment Agreement or any other agreement with Neurogene, subject to a 30-day cure period or (vi) failure to comply in any material respect with applicable laws with respect to the operation of the business of Neurogene, subject to a 30-day cure period.
- "Change in Control" generally means (i) a merger or consolidation of Neurogene with or into any other corporation or other entity or person, (ii) a sale, lease, exchange, or other transfer in one transaction or a series of related transactions of all or substantially all Neurogene's assets to an unrelated person or entity, or (iii) any other transaction, including the sale by Neurogene of shares of Neurogene capital stock or a transfer of existing shares of Neurogene capital stock which results in a third party that is not an affiliate of Neurogene or its stockholders (or a group of third parties that are not affiliates of Neurogene or its stockholders) immediately prior to such transaction acquires or holds Neurogene capital stock representing a majority of Neurogene's outstanding voting power immediately following such transaction.
- "Good Reason" generally means (i) a material diminution in annual base salary (excluding across the board reductions), (ii) any material diminution in title, authority, responsibilities or lines of reporting, or (iii) a required geographic relocation by more than 50 miles, in each case, subject to standard notice and cure periods.

On April 1, 2024, we entered into the Mikail Employment Agreement pursuant to which Ms. Mikail is entitled to receive the following severance amounts upon termination by the Company for "cause" or by Ms. Mikail for "good reason" subject to Ms. Mikail's execution and non-revocation of a release of claims in favor of the Company and continued compliance with certain restrictive covenants:

- a lump sum payment equal to 12 months of her annual base salary then in effect;
- any bonus earned for the fiscal year prior to such termination but not yet paid;
- a pro rata annual bonus amount calculated based on the number of days from and including the first day of the then-current fiscal year to the date of termination; and
- premium payments for up to 12 months of continuing healthcare coverage benefits for Ms. Mikail and any dependents who are covered by her healthcare benefits.

If Ms. Mikail is terminated by the Company without "cause" or resigns for "good reason" during a period that is three months prior to or twelve months following a change in control (as that term is defined in the 2023 Plan):

- a lump sum payment equal to 1.25x her base salary and target bonus then in effect;
- any bonus earned for the fiscal year prior to such termination but not yet paid;
- up to 18 months of continuing health care coverage benefits for Ms. Mikail and any dependents who are covered by her health care benefits; and
- acceleration of the vesting of all equity and equity-based awards held by Ms. Mikail as of such termination.

For purposes of the Mikail Employment Agreement:

- “Cause” generally means a good faith determination by the Board that her employment be terminated due to her indictment, conviction or plea to a felony or other crime involving fraud, dishonesty or moral turpitude, material misconduct or gross negligence in performing her duties, her material failure or refusal to follow policies and lawful directives of the Board, any fraud, embezzlement, theft or dishonesty in connection with her employment by the Company, her material breach of the Mikail Employment Agreement or her failure to comply in any material respect with applicable laws with respect to the operation of the business.
- “Good Reason” generally means the failure or refusal of the Company to comply with the terms of the Mikail Employment Agreement in any material respect, a material diminution in Ms. Mikail’s duties, title, authority, status or responsibilities, a material reduction in her base salary or annual cash bonus opportunity (in each case unless it is part of a reduction that applies similarly and in substantially the same way to similarly situated officers), or requiring Ms. Mikail to be located at an office that is more than 50 miles from the Company’s current headquarters (other than pursuant to a stay home order from a governmental entity), in each case, subject to standard notice and cure periods.
- “Change in Control” generally means any person becoming the beneficial owner of more than 50% of the securities of the Company (directly or indirectly), a change in the majority of the Board of Directors, consummation of a merger or other acquisition pursuant to which the beneficial owners of the Company prior to such transaction no longer hold a majority of the voting securities of the Company following such transaction, the implementation of a plan of dissolution or liquidation, or the consummation of a sale of more than 50% of the Company’s assets to an entity whose beneficial ownership is less than 50% owned by the prior beneficial owners of the Company in substantially the same proportions as before such transaction.

*Stuart Cobb*

On April 19, 2024, we entered into the Restated Consulting Agreement pursuant to which Dr. Cobb provides services to the Company as our Chief Scientific Officer. Pursuant to the Restated Consulting Agreement, the Consultant will be entitled to receive the following payments upon a termination by the Company without “cause” or by the Consultant with “good reason” (in each case as defined in the Restated Consulting Agreement) subject to the Consultant’s execution and non-revocation of a release of claims in favor of the Company and continued compliance with certain restrictive covenants:

- a lump sum payment equal to 1.0x the then-current annual consulting fee;
- any annual success fee earned for the fiscal year prior to such termination but not yet paid; and
- a pro rata amount of the annual success fee for the then-current year calculated based on the number of days from and including the first day of the fiscal year to the date of termination.

If the Consultant is terminated by the Company without “cause” or if the Consultant terminates with “good reason” within a period from three months before to 12 months after a change in control, then in lieu of the foregoing, the Consultant will be entitled to receive:

- a lump sum payment equal to 12 months of the then-current consulting fee;
- any annual success fee earned for the fiscal year prior to such termination but not yet paid;
- a pro rata amount of the annual success fee for the then-current year calculated based on the then-current target annual success fee and the number of days from and including the first day of the fiscal year to the date of termination; and
- acceleration of the vesting of all equity and equity-based awards held by the Consultant as of such termination.

For purposes of the Restated Consulting Agreement:

- “Cause” generally means a good faith determination by the Board that Dr. Cobb’s consulting arrangement be terminated due to his indictment, conviction or plea to a felony or other crime involving fraud, dishonesty or moral turpitude, material misconduct or gross negligence in performing his duties, his material failure or refusal to follow policies and lawful directives of the Board, any fraud, embezzlement, theft or dishonesty in connection with his consulting services provided to the Company, his material breach of the Restated Consulting Agreement or his failure to comply in any material respect with applicable laws with respect to the operation of the business.
- “Good Reason” generally means the failure or refusal of the Company to comply with the terms of the Restated Consulting Agreement in any material respect, a material diminution in Dr. Cobb’s responsibilities, a material reduction in his base salary or annual success fee opportunity, or requiring Dr. Cobb to be located at an office that is more than 50 miles from his current work location.
- “Change in Control” generally means any person becoming the beneficial owner of more than 50% of the securities of the Company (directly or indirectly), a change in the majority of the Board of Directors, consummation of a merger or other acquisition pursuant to which the beneficial owners of the Company prior to such transaction no longer hold a majority of the voting securities of the Company following such transaction, the implementation of a plan of dissolution or liquidation, or the consummation of a sale of more than 50% of the Company’s assets to an entity whose beneficial ownership is less than 50% owned by the prior beneficial owners of the Company in substantially the same proportions as before such transaction.

## **Director Compensation**

### ***Non-Employee Director Compensation Policy***

Prior to the Closing, our non-employee directors received the following annual cash compensation for service on our Board and the following additional annual cash compensation for services on committees of our Board, as applicable, payable in equal monthly installments, in arrears:

- \$40,000 per year for service as a member of our Board;
- \$25,000 per year for service as our Chairperson;
- \$20,000 per year for service as the chair of the Audit Committee and \$8,000 per year for service as a member (other than as the chair) of the Audit Committee;
- \$15,000 per year for service as the chair of the Compensation Committee and \$7,000 per year for service as a member (other than as the chair) of the Compensation Committee; and
- \$8,000 per year for service as the chair of the Nominating Committee and \$5,000 per year for service as a member (other than as the chair) of the Nominating Committee.
- We have a policy of reimbursing our directors for their reasonable out-of-pocket expenses incurred in attending Board and committee meetings. Employee directors do not receive additional compensation for service on our Board.
- In addition to the cash compensation set forth above, prior to the Closing, each new non-employee director who joined our Board was granted an option to purchase 2,500 shares of our common stock (after giving effect to the 1-for-5 Reverse Stock Split and the 1-for-4 Reverse Stock Split). These options vest in equal annual installments over a three-year period. Thereafter, each non-employee director continuing in office following an annual meeting received a grant of stock options covering 1,250 shares of our common stock (after giving effect to the 1-for-5 Reverse Stock Split and the 1-for-4 Reverse Stock Split), vesting in equal monthly installments over a one-year period following the date of such annual meeting.

On January 18, 2024, we adopted a policy for compensating our non-employee directors with a combination of cash and equity, with such equity awards being subject to the terms and conditions of our 2023 Plan, and the Stock Option Agreement thereunder and related forms of grant notices approved by the Board.

- **Cash Compensation.** Each of our non-employee directors is eligible to receive a \$40,000 annual cash retainer for serving as a member of the Board (except for a non-executive Chair of the Board, if one is appointed, who would be eligible to receive a \$70,000 annual cash retainer in such a position) as well as the following additional annual cash fees for their committee service:

	Chair	Member
Audit Committee	\$ 15,000	\$ 7,500
Compensation Committee	\$ 10,000	\$ 5,000
Nominating Committee	\$ 8,000	\$ 4,000

Each annual cash retainer and additional annual fee is paid quarterly in arrears. In addition, we reimburse all of our directors for their reasonable out-of-pocket expenses, including travel, food and lodging, incurred by them in connection with attendance at Board and committee meetings.

- **Equity Compensation.** New non-employee directors are entitled to receive an initial equity grant of 15,400 stock options. Subject to the director's continued service, initial equity awards vest in equal monthly installments over a three-year period following the date of grant. In addition, each non-employee director is entitled to receive an annual equity grant of 7,700 stock options. The annual equity awards vest in full on the first to occur of the first anniversary of the date of grant or the next annual meeting of the Company's stockholders, subject to the director's continued service through such date. All non-employee directors received an initial equity grant of 15,400 stock option awards in January 2024.

#### ***Fiscal Year 2023 Non-Employee Director Compensation Table***

The following table shows the compensation earned in 2023 by the non-employee directors who served on the Board during such year. Amounts received by Dr. Baffi in 2023 relate to his service on the board of Neurogene OpCo. Dr. Freedland and Mr. Woods joined the Board of Directors in connection with the Closing in December 2023 and as such did not receive any compensation in 2023.

Name	Fees Earned or Paid in Cash (\$)	Option Awards (\$) <sup>(1)</sup>	All Other Compensation (\$)	Total (\$)
<b>Non-Employee Directors After the Reverse Merger</b>				
Robert Baffi	30,000	34,081	—	64,081
Cory Freedland	—	—	—	—
Sarah Noonberg	48,000	14,124	—	62,124
Rohan Palekar	57,500	14,124	—	71,624
Keith Woods	—	—	—	—
<b>Non-Employee Directors Prior to the Reverse Merger<sup>(2)</sup></b>				
Martin Babler	53,057	14,124	—	67,181
M. Cantey Boyd	50,163	14,124	—	64,287
Erin Lavelle	51,128	14,124	—	65,252
Todd Simpson	81,997	14,124	—	96,121

(1) The amounts in this column represent the aggregate grant date fair value of the stock options granted to each non-employee director during the 2023 fiscal year under Neoleukin's 2014 Plan with respect to Dr. Noonberg, Mr. Palekar, Mr. Babler, Ms. Boyd, Ms. Lavelle and Mr. Simpson and our 2018 Plan with respect to Dr. Baffi, computed in accordance with FASB ASC Topic 718. For more information regarding the assumptions used in these calculations, see Note 14 to our consolidated financial statements, Stock-Based Compensation, in our Annual Report on Form 10-K for the year ended December 31, 2023.

(2) These directors resigned from the Board effective as of December 18, 2023 in connection with the Reverse Merger.

Dr. McMinn, our CEO and Executive Chair, and Dr. Drachman, our former Chief Executive Officer and President, did not receive any additional compensation for his or her service on the Board. The compensation received by Dr. McMinn and Dr. Drachman for their respective service as our CEO is presented in the 2023 Summary Compensation Table above.

As of December 31, 2023, our non-employee directors held the following options to purchase shares of our common stock:

Name	Number of Shares Subject to Outstanding Options as of December 31, 2023 (#)
Robert Baffi	11,642
Sarah B. Noonberg	6,100
Rohan Palekar	5,000
Martin Babler	6,250
M. Cantey Boyd	6,100
Erin Lavelle	6,250
Todd Simpson	8,341

## SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

The following table sets forth information, to the extent known by us or ascertainable from public filings, with respect to the beneficial ownership of our Common Stock as of November 13, 2024 by:

- each of our directors;
- each of our Named Executive Officers;
- all of our directors and executive officers as a group; and
- each person, or group of affiliated persons, who is known by us to be the beneficial owners of more than 5.0% of our Common Stock.

Beneficial ownership is determined in accordance with the rules of the SEC, and thus represents voting or investment power with respect to our securities. Under such rules, beneficial ownership includes any shares over which the individual has sole or shared voting power or investment power as well as any shares that the individual has the right to acquire within 60 days after the date of this table. To our knowledge and subject to applicable community property rules, the persons and entities named in the table have sole voting and sole investment power with respect to all equity interests beneficially owned, unless otherwise indicated.

The percentage ownership information shown in the column titled “Percentage of Shares Beneficially Owned” in the table below is based on 14,854,725 shares of our common stock outstanding as of the date of this table (plus any shares that such person has the right to acquire within 60 days after the date of this table). Unless otherwise indicated, the address of each individual listed, in this table is c/o Neurogene Inc., 535 W. 24th Street, 5th Floor, New York, NY 10011.

Name of Beneficial Owner	Beneficial Ownership	
	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned
<b>Greater than 5% Stockholders:</b>		
Entities affiliated with EcoR1 Capital, LLC <sup>(1)</sup>	1,507,588	9.99 %
Entities affiliated with Redmile Group, LLC <sup>(2)</sup>	1,505,861	9.99 %
Entities affiliated with RTW Investments, LP <sup>(3)</sup>	1,498,829	9.99 %
Samsara BioCapital, L.P. <sup>(4)</sup>	1,456,629	9.80 %
Entities affiliated with BlackRock, Inc. <sup>(5)</sup>	1,394,714	9.39 %
Entities affiliated with Casdin Capital, LLC <sup>(6)</sup>	1,024,616	6.90 %
<b>Named Executive Officers and Directors:</b>		
Rachel McMinn <sup>(7)</sup>	1,293,940	8.69 %
Christine Mikail <sup>(8)</sup>	85,189	*
Stuart Cobb <sup>(8)</sup>	59,059	*
Donna Cochener <sup>(9)</sup>	25,198	*
Jonathan Drachman <sup>(10)</sup>	114,503	*
Sean Smith <sup>(8)</sup>	7,247	*
Robert Baffi <sup>(8)</sup>	15,062	*
Cory Freedland <sup>(11)</sup>	4,705	*
Rohan Palekar <sup>(8)</sup>	9,705	*
Sarah Noonberg <sup>(8)</sup>	10,805	*
R. Keith Woods <sup>(8)</sup>	4,705	*
All current executive officers and directors as a group (9 persons) <sup>(12)</sup>	1,483,170	9.96 %

\* Represents beneficial ownership of less than one percent.

- (1) Based on the Company's records. Consists of (i) 1,100,388 shares of Common Stock held by EcoR1 Capital Fund Qualified, L.P. ("Qualified Fund"), (ii) 85,993 shares of Common Stock held by EcoR1 Capital Fund, L.P. ("Capital Fund"), (iii) 84,961 shares of Common Stock held by EcoR1 Venture Opportunity Fund, L.P. ("Opportunity Fund") and (iv) 236,246 shares of Common Stock issuable in the aggregate upon the exercise of Pre-Funded Warrants held by Qualified Fund and Capital Fund. Pursuant to the terms of the Pre-Funded Warrants, the Pre-Funded Warrants will be exercisable only to the extent that after giving effect to such exercise the holder thereof and their affiliates would beneficially own no more than 9.99% of outstanding Common Stock of the Company. However, Qualified Fund or Capital Fund may from time to time provide written notice to the Company to increase such beneficial ownership limitation to any other percentage not in excess of 19.99%. Any such change will not be effective until the 61st day after such notice is delivered to the Company. As a result of this restriction, the number of shares of Common Stock that may be issued upon exercise of the Pre-Funded Warrants by Qualified Fund or Capital Fund may change depending upon changes in the outstanding shares of Common Stock. Without giving effect to the above beneficial ownership limitation, the Pre-Funded Warrants that Qualified Fund holds would be exercisable for an aggregate of 1,041,172 shares of Common Stock and the Pre-Funded Warrants that Capital Fund holds would be exercisable for an aggregate of 48,486 shares of Common Stock. EcoR1 Capital, LLC ("EcoR1") is the general partner of Qualified Fund and Capital Fund and the investment advisor to Opportunity Fund. Biotech Opportunity GP, LLC ("Opportunity GP") is the general partner of Opportunity Fund. Oleg Nodelman is the control person of EcoR1 and Opportunity GP and may be deemed to share dispositive and voting power over the shares held by Qualified Fund, Capital Fund and Opportunity Fund. Mr. Nodelman, EcoR1 and Opportunity GP each disclaim beneficial ownership of all shares except to the extent of their pecuniary interest therein. The address of the above persons is 357 Tehama Street #3, San Francisco, CA 94103.
- (2) Based on information provided in a Schedule 13G/A filed with the SEC on February 14, 2024 and, with respect to certain securities, the Company's records. Consists of 1,286,897 shares of Common Stock and 218,964 shares of Common Stock issuable upon the exercise of Pre-Funded Warrants held by certain private investment vehicles (the "Redmile Funds") managed by Redmile Group, LLC ("Redmile"), including 652,030 shares of Common Stock held by Redmile Biopharma Investments I, L.P. ("Redmile Biopharma I"). Pursuant to the terms of the Pre-Funded Warrants, the Pre-Funded Warrants will be exercisable only to the extent that after giving effect to such exercise the holder thereof and their affiliates would beneficially own no more than 9.99% of outstanding Common Stock of the Company. However, Redmile may from time to time provide written notice to the Company, to increase such beneficial ownership limitation to any other percentage not in excess of 19.99%. Any such change will not be effective until the 61st day after such notice is delivered to the Company. As a result of this restriction, the number of shares of Common Stock that may be issued upon exercise of the Pre-Funded Warrants by Redmile Funds may change depending upon changes in the outstanding shares of Common Stock. Without giving effect to the above beneficial ownership limitation, the Pre-Funded Warrants that Redmile Funds hold would be exercisable for an aggregate of 263,410 shares of Common Stock, including 160,003 shares of Common Stock issuable upon the exercise of Pre-Funded Warrants held by Redmile Biopharma I. Redmile is the investment manager/adviser to the Redmile Funds, and, in such capacity, exercises voting and investment power over all of the securities held by the Redmile Funds and may be deemed to be the beneficial owner of these securities. Jeremy C.

- Green serves as the managing member of Redmile and also may be deemed to be the beneficial owner of these securities. Redmile and Mr. Green each disclaim beneficial ownership of these securities, except to the extent of its or his pecuniary interest in such securities, if any. The address of the Redmile Funds is c/o Redmile Group, LLC, One Letterman Drive, Building D, Suite D3-300, San Francisco, CA 94129.
- (3) Based on information provided in a Schedule 13G filed with the SEC on November 14, 2024 and, with respect to certain securities, the Company's records. Consists of 1,350,256 shares of Common Stock and 148,573 shares of Common Stock issuable upon the exercise of Pre-Funded Warrants held in the aggregate by RTW Master Fund, Ltd., RTW Innovation Master Fund, Ltd. and RTW Biotech Opportunities Operating Ltd. (collectively, the "RTW Funds"). Pursuant to the terms of the Pre-Funded Warrants, the Pre-Funded Warrants will be exercisable only to the extent that after giving effect to such exercise the holder thereof and their affiliates would beneficially own no more than 9.99% of outstanding Common Stock of the Company. However, RTW Investments, LP ("RTW") may from time to time provide written notice to the Company, to increase such beneficial ownership limitation to any other percentage not in excess of 19.99%. Any such change will not be effective until the 61st day after such notice is delivered to the Company. As a result of this restriction, the number of shares of Common Stock that may be issued upon exercise of the Pre-Funded Warrants by RTW Funds may change depending upon changes in the outstanding shares of Common Stock. Without giving effect to the above beneficial ownership limitation, the Pre-Funded Warrants that the RTW Funds hold would be exercisable for an aggregate of 385,007 shares of Common Stock. RTW, in its capacity as the investment manager of the RTW Funds, has the power to vote and the power to direct the disposition of the shares held by the RTW Funds. Accordingly, RTW may be deemed to be the beneficial owner of such securities. Roderick Wong, M.D., as the Managing Partner of RTW, has the power to direct the vote and disposition of the securities held by RTW. Dr. Wong disclaims beneficial ownership of the shares held by the RTW Funds, except to the extent of his pecuniary interest therein. The address and principal office of RTW Investments, LP is 40 10th Avenue, Floor 7, New York, NY 10014, and the address of Dr. Wong and each of the RTW Funds is c/o RTW Investments, LP, 40 10th Avenue, Floor 7, New York, NY 10014.
  - (4) Based on information provided in a Schedule 13G filed with the SEC on February 14, 2024 and, with respect to certain securities, the Company's records. Consists of (i) 1,447,790 shares of Common Stock, (ii) 4,705 shares of Common Stock issuable upon exercise of stock options held by Cory Freedland exercisable within 60 days of the date of this table and (iii) 4,134 shares of Common Stock issuable upon the exercise of Pre-Funded Warrants held by Samsara BioCapital, L.P. ("Samsara LP"). Pursuant to the terms of the Pre-Funded Warrants, the Pre-Funded Warrants will be exercisable only to the extent that after giving effect to such exercise the holder thereof and their affiliates would beneficially own no more than 9.80% of outstanding Common Stock of the Company. However, Samsara LP may from time to time provide written notice to the Company, to increase such beneficial ownership limitation to any other percentage not in excess of 19.99%. Any such change will not be effective until the 61st day after such notice is delivered to the Company. As a result of this restriction, the number of shares of Common Stock that may be issued upon exercise of the Pre-Funded Warrants by Samsara may change depending upon changes in the outstanding shares of Common Stock. Without giving effect to the above beneficial ownership limitation, the Pre-Funded Warrants that Samsara holds would be exercisable for an aggregate of 87,070 shares of Common Stock. Samsara BioCapital GP, LLC ("Samsara GP") is the sole general partner of Samsara LP and may be deemed to have voting and investment power over the securities held by Samsara LP. Dr. Srinivas Akkaraju is a managing member of Samsara GP and may be deemed to have voting and dispositive power over the securities held by Samsara LP. The address of the above persons is 628 Middlefield Road, Palo Alto, CA 94301.
  - (5) Based on information provided in a Schedule 13G filed with the SEC on July 8, 2024, which reported that BlackRock, Inc. ("BlackRock") held sole voting power over 1,385,699 shares of Common Stock, shared voting power over 0 shares of Common Stock, sole dispositive power over 1,394,714 shares and shared dispositive power over 0 shares of Common Stock. The address for BlackRock is 50 Hudson Yards, New York, NY 10001.
  - (6) Based on information provided in a Schedule 13G filed with the SEC on November 8, 2024 and, with respect to certain securities, the Company's records. Consists of (i) 804,255 shares of Common Stock held by Casdin Partners Master Fund, L.P. ("CPM Fund"), (ii) 132,217 shares of Common Stock held by Casdin Private Growth Equity Fund, L.P. ("CPGE Fund") and (iii) 88,144 shares of Common Stock held by Casdin Private Growth Equity Fund II, L.P. ("CPGE II Fund"). Casdin Partners GP, LLC ("CP GP") is the general partner of CPM Fund. Casdin Private Growth Equity Fund GP, LLC ("CPGE GP") is the general partner of CPGE Fund, Casdin Private Growth Equity Fund II GP, LLC ("CPGE II GP") is the general partner of CPGE II Fund, and Casdin Capital, LLC ("Casdin Capital") is the investment adviser to each of CPM, CPGE Fund and CPGE II Fund. Eli Casdin is the Managing Member of Casdin Capital, CP GP, CPGE GP and CPGE II GP. Mr. Casdin, Casdin Capital, CP GP, CPGE GP and CPGE II GP disclaim beneficial ownership of any of the reported shares of Common Stock herein, except to the extent of their pecuniary interest therein. The address of the foregoing persons is 1350 Avenue of the Americas, Suite 2600, New York, NY 10019.
  - (7) Includes options to purchase 36,494 shares of Common Stock that are exercisable within 60 days of the date of this table.
  - (8) Consists of options to purchase shares of Common Stock that are exercisable within 60 days of the date of this table.
  - (9) Includes options to purchase 24,998 shares of Common Stock that are exercisable within 60 days of the date of this table.
  - (10) Consists of (i) 94,503 shares held directly by Dr. Drachman, (ii) 10,000 shares held by the JGD Family Trust 2020, and (iii) 10,000 shares held by the PLD Family Trust 2020.
  - (11) Consists of options to purchase shares of Common Stock exercisable within 60 days of the date of this table. Dr. Freedland, an employee of Samsara LP, serves on our Board as a representative of Samsara LP, and Samsara LP may be deemed to beneficially own the securities received by Dr. Freedland as compensation for serving as a director. Pursuant to the policies of Samsara LP, Dr. Freedland does not have any right to the pecuniary interest in securities received as compensation for serving as a director and Samsara LP is entitled to an indirect pecuniary interest in such securities.
  - (12) Includes options to purchase 225,724 shares of Common Stock that are exercisable within 60 days of the date of this table.



## CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS

The following is a summary of each transaction or series of similar transactions since January 1, 2021, or any currently proposed transaction, to which we were or are a party in which:

- the amount involved exceeds \$120,000; and
- any related person (including our directors, executive officers, beneficial owners of more than 5% of our voting capital stock and any members of their immediate family) had or will have a direct or indirect material interest, other than compensation (including the consulting agreement relating to Dr. Cobb’s provision of services to the Company as Chief Scientific Officer) and other arrangements that are described under the section titled “Executive Compensation” or that were approved by our Compensation Committee.

Beneficial ownership of securities is determined in accordance with the rules of the SEC.

### Related Party Transactions

#### *2024 Private Placement*

On November 5, 2024, Neurogene completed the 2024 Private Placement. Pursuant to the Securities Purchase Agreement, Neurogene issued and sold, and the investors purchased, (i) 1,835,000 shares of Common Stock and (ii) 2,165,042 2024 Pre-Funded Warrants, exercisable for 2,165,042 shares of Common Stock, at a purchase price of \$50.00 per share or \$49.999 per 2024 Pre-Funded Warrant, which represents the per share purchase price of the Common Stock less the \$0.001 per share exercise price for each 2024 Pre-Funded Warrant, for an aggregate purchase price of approximately \$200.0 million. Six of the investors or their affiliates were or became upon completion of the transaction beneficial holders of more than 5% of Common Stock, and the table below sets forth the number of shares of Common Stock or 2024 Pre-Funded Warrants purchased by such holders at the closing of the 2024 Private Placement.

Participant	Shares of Common Stock	2024 Pre-Funded Warrants	Total Purchase Price
Entities affiliated with RTW Investments, LP	21,500	385,007	\$ 30,000
Entities affiliated with Casdin Capital, LLC	50,000	—	\$ 25,000
Entities affiliated with Redmile Group, LLC	34,000	160,003	\$ 25,000
Entities affiliated with EcoR1	—	50,001	\$ 25,000
Entities affiliated with Great Point Partners	36,000	—	\$ 18,000
Entities affiliated with Samsara BioCapital, LP	18,000	2,000	\$ 10,000

#### *2024 Registration Rights Agreement*

On November 5, 2024, the Company entered into the 2024 Registration Rights Agreement with the Selling Stockholders named therein pursuant to which, among other things, the Company agreed to provide for the registration and resale of certain shares of Common Stock that are held by such Selling Stockholders from time to time, including the shares of Common Stock issuable upon exercise of the 2024 Pre-Funded Warrants. See the section titled “*Description of Capital Stock—Registration Rights Agreements*” located elsewhere in this prospectus for further information on the 2024 Registration Rights Agreement.

#### *Agreements with Baker Bros Advisors LP and affiliates*

Prior to the Reverse Merger, entities affiliated with Baker Bros. Advisors LP (“BBA”), beneficially owned more than 5% of our outstanding Common Stock. BBA is a party to the 2016 Registration Rights agreement described below under “*Selling Stockholders — Registration Rights Agreements*”. The Company also entered into a

Letter Agreement with BBA on July 17, 2023, pursuant to which BBA has the right to nominate one person for election as a director of the Company at any time that BBA owns at least 12.5% of the then-outstanding stock of the Company and which also obligates the Company to enter into a Registration Rights Agreement with any BBA entity who may be deemed an “affiliate” of the Company.

#### ***Indemnification Agreements and Insurance***

Our Bylaws require us to indemnify our directors and officers to the fullest extent permitted by Delaware law. We have also entered into agreements to indemnify our directors and executive officers. These agreements, among other things, require us to indemnify these individuals for certain expenses (including attorneys’ fees), judgments, fines and settlement amounts reasonably incurred by such person in any action or proceeding, including any action by or in our right, on account of any services undertaken by such person on behalf of the Company or that person’s status as a director or officer, as applicable, to the maximum extent allowed under Delaware law. We have also purchased directors’ and officers’ liability insurance.

#### **Review, Approval or Ratification of Transactions with Related Parties**

Our Board has a written policy regarding the review and approval or ratification by our Audit Committee of related person transactions. For purposes of our policy only, a related person transaction is a transaction, arrangement or relationship or any series of similar transactions, arrangements or relationships between us or any of our subsidiaries and any related person in which the aggregate amount involved since the beginning of our last completed fiscal year exceeds or is expected to exceed \$120,000 and such related person has or will have a direct or indirect interest. A related person is defined to include any executive officers, directors or director nominees or beneficial owner of more than 5% of our common stock and any immediate family member of any of the foregoing persons. In determining to approve or ratify any such transaction, our Audit Committee is expected to take into account, among other factors it deems appropriate, whether the transaction is on terms no less favorable than terms generally available to an unaffiliated third-party under the same or similar circumstances and the extent of the related person’s interest in the transaction. Transactions involving compensation for services provided to us as an employee or director, among other limited exceptions, are deemed under the terms of the policy to have standing pre-approval by the Audit Committee but may be specifically reviewed if appropriate in light of the facts and circumstances. Any director who is a related person with respect to a transaction under review is not permitted to participate in the deliberations (other than to provide information concerning the transaction to the Audit Committee) or vote on approval of the transaction.

## SELLING STOCKHOLDERS

This prospectus covers the resale or other disposition from time to time by the Selling Stockholders identified in the table below of up to an aggregate of 4,000,042 shares of our Common Stock. The Selling Stockholders may from time to time offer and sell any or all of the Resale Shares set forth below pursuant to this prospectus and any accompanying prospectus supplement.

On November 4, 2024, we entered into the SPA, pursuant to which we issued and sold (i) an aggregate of 1,835,000 shares of Common Stock, at a purchase price of \$50.00 per share, and (ii) an aggregate of 2,165,042 Pre-Funded Warrants to purchase an aggregate of 2,165,042 shares of our Common Stock at a purchase price of \$49.999 per Pre-Funded Warrant, which represents the per share purchase price of the Private Placement Common Shares less the \$0.001 per share exercise price for each Pre-Funded Warrant, at an aggregate purchase price of approximately \$200.0 million.

Concurrent with the closing of PIPE, we entered into the RRA with the Selling Stockholders, pursuant to which we are obligated to file a resale registration statement covering the securities described herein. The registration statement of which this prospectus is a part is being filed pursuant to the RRA.

This prospectus covers the resale or other disposition by the Selling Stockholders or their pledgees, donees, transferees or other successors-in-interest that receive their shares after the date of this prospectus of up to the total number of shares of Resale Shares. Throughout this prospectus, when we refer to the “Selling Stockholders,” we are referring to the purchasers under the SPA listed in the table below.

We are registering the resale of the Resale Shares to permit the Selling Stockholders and their pledgees, donees, transferees or other successors-in interest that receive their shares after the date of this prospectus to resell or otherwise dispose of the shares in the manner contemplated under “Plan of Distribution” herein.

Cory Freedland, PhD, is a partner of Samsara LP and a member of our board of directors. In addition, Robert Baffi is a Venture Partner at Samsara and a member of our board of directors. From December 2019 until the closing of the Reverse Merger, M. Cantey Boyd also served as a director of the Company as a representative of Baker Funds (as defined below) and their affiliates. The Company also entered into a Letter Agreement with Baker Bros. Advisors LP (“BBA”) on July 17, 2023, pursuant to which BBA has the right to nominate one person for election as a director of the Company at any time that BBA owns at least 12.5% of the then-outstanding stock of the Company and which also obligates the Company to enter into a Registration Rights Agreement with any BBA entity who may be deemed an “affiliate” of the Company. Eli Casdin is the Managing Member of Casdin Capital, CP GP, CPGE GP and CPGE II GP, and served as a director of the Company from February 2021 until October 2022. Except as otherwise disclosed herein, the Selling Stockholders do not have, and within the past three years have not had, any position, office or other material relationship with us.

The following table sets forth the names of the Selling Stockholders, the number of shares of Common Stock beneficially owned by the Selling Stockholders as of November 13, 2024, the number of shares of Common Stock that may be offered under this prospectus and the number of shares of our Common Stock that will be owned after this offering by the Selling Stockholders assuming all of the shares registered for resale hereby are sold.

The Selling Stockholders may sell some, all or none of their Resale Shares. We do not know how long the Selling Stockholders will hold the Resale Shares before selling them, and we currently have no agreements, arrangements or understandings with the Selling Stockholders regarding the sale or other disposition of any of the Resale Shares.

The information set forth below is based upon information obtained from the Selling Stockholders and upon information in our possession regarding the issuance of the shares of Common Stock and Pre-Funded Warrants in

connection with the PIPE. The percentages of Common Stock beneficially owned after the offering by each Selling Stockholder below are based on 14,854,725 shares of Common Stock outstanding as of November 13, 2024.

Name of Selling Stockholders <sup>(1)</sup>	Common Stock Beneficially Owned Before Offering <sup>(2)</sup>	Common Stock that May Be Offered Pursuant to Prospectus	Common Stock Beneficially Owned After Offering <sup>(2)</sup>	
			Number	Percentage (%)
Entities affiliated with RTW Investments, LP <sup>(3)</sup>	1,498,829	600,007	1,135,256	7.45
Samsara BioCapital, LP <sup>(4)</sup>	1,456,629	200,000	1,339,565	8.96
Entities affiliated with Great Point Partners, LLC <sup>(5)</sup>	464,019	360,000	104,019	*
Entities affiliated with EcoR1 Capital, LLC <sup>(6)</sup>	1,507,588	500,010	1,563,082	9.99
Entities affiliated with Redmile Group, LLC <sup>(7)</sup>	1,505,861	500,003	1,050,304	6.95
Entities affiliated with Casdin Capital LLC <sup>(8)</sup>	1,024,616	500,000	524,616	3.53
Entities affiliated with Baker Bros. Advisors LP <sup>(9)</sup>	756,362	1,100,022	814,136	4.99
Commodore Capital Master LP <sup>(10)</sup>	240,000	240,000	—	—

\* Less than 1%

- (1) To our knowledge, unless otherwise indicated, all persons named in the table above have sole voting and investment power with respect to their shares of Common Stock.
- (2) “Beneficial ownership” is a term broadly defined by the SEC in Rule 13d-3 under the Exchange Act, and includes more than the typical form of stock ownership, that is, stock held in the person’s name. The term also includes what is referred to as “indirect ownership,” meaning ownership of shares as to which a person has or shares investment power.
- (3) Shares listed under “Common Stock Beneficially Owned Before the Offering” consist of 1,350,256 shares of Common Stock and 148,573 shares of Common Stock issuable upon the exercise of Pre-Funded Warrants held in the aggregate by RTW Funds. Pursuant to the terms of the Pre-Funded Warrants, the Pre-Funded Warrants will be exercisable only to the extent that after giving effect to such exercise the holder thereof and their affiliates would beneficially own no more than 9.99% of outstanding Common Stock of the Company. However, RTW may from time to time provide written notice to the Company, to increase such beneficial ownership limitation to any other percentage not in excess of 19.99%. Any such change will not be effective until the 61st day after such notice is delivered to the Company. As a result of this restriction, the number of shares of Common Stock that may be issued upon exercise of the Pre-Funded Warrants by RTW Funds may change depending upon changes in the outstanding shares of Common Stock. Without giving effect to the above beneficial ownership limitation, the Pre-Funded Warrants that the RTW Funds hold would be exercisable for an aggregate of 385,007 shares of Common Stock. RTW, in its capacity as the investment manager of the RTW Funds, has the power to vote and the power to direct the disposition of the shares held by the RTW Funds. Accordingly, RTW may be deemed to be the beneficial owner of such securities. Roderick Wong, M.D., as the Managing Partner of RTW, has the power to direct the vote and disposition of the securities held by RTW. Dr. Wong disclaims beneficial ownership of the shares held by the RTW Funds, except to the extent of his pecuniary interest therein. The address and principal office of RTW Investments, LP is 40 10th Avenue, Floor 7, New York, NY 10014, and the address of Dr. Wong and each of the RTW Funds is c/o RTW Investments, LP, 40 10th Avenue, Floor 7, New York, NY 10014.
- (4) Shares listed under “Common Stock Beneficially Owned Before the Offering” consist of (i) 1,447,790 shares of Common Stock, (ii) 4,705 shares of Common Stock issuable upon exercise of stock options held by Cory Freedland exercisable within 60 days of the date of this table and (iii) 4,134 shares of Common Stock issuable upon the exercise of Pre-Funded Warrants held by Samsara LP. Pursuant to the terms of the Pre-Funded Warrants, the Pre-Funded Warrants will be exercisable only to the extent that after giving effect to such exercise the holder thereof and their affiliates would beneficially own no more than 9.80% of outstanding Common Stock of the Company. However, Samsara LP may from time to time provide written notice to the Company, to increase such beneficial ownership limitation to any other percentage not in excess of 19.99%. Any such change will not be effective until the 61st day after such notice is delivered to the Company. As a result of this restriction, the number of shares of Common Stock that may be issued upon exercise of the Pre-Funded Warrants by Samsara may change depending upon changes in the outstanding shares of Common Stock. Without giving effect to the above beneficial ownership limitation, the Pre-Funded Warrants that Samsara holds would be exercisable for an aggregate of 87,070 shares of Common Stock. Samsara GP is the sole general partner of Samsara LP and may be deemed to have voting and investment power over the securities held by Samsara LP. Dr. Srinivas Akkaraju is a managing member of Samsara GP and may be deemed to have voting and dispositive power over the securities held by Samsara LP. The address of the above persons is 628 Middlefield Road, Palo Alto, CA 94301.
- (5) Shares listed under “Common Stock Beneficially Owned Before the Offering” consist of (i) 301,612 shares of Common Stock held by Biomedical Value Fund, L.P. (“BVF”) and (ii) 162,407 shares of Common Stock held by Biomedical Offshore Value Fund, Ltd. (“BOVF”) and together with BVF, the “GPP Entities”). Great Point Partners, LLC (“Great Point”) is the investment manager of the GPP Entities and by virtue of such status may be deemed to be the beneficial owner of the shares held by each of the GPP Entities. Each of Dr. Jeffrey R. Jay, M.D., as Senior Managing Member of Great Point, and Ms. Lillian Nordahl, as Managing Director of Great Point, has voting and investment power with respect to the shares held by each of the GPP Entities, and therefore may be deemed to be the beneficial owner of the shares held by the GPP Entities, except to the extent of their respective pecuniary interests. The address for the foregoing persons is 165 Mason Street, 3rd Floor, Greenwich, CT 06830.

- (6) Shares listed under “Common Stock Beneficially Owned Before the Offering” consist of (i) 1,100,388 shares of Common Stock held by Qualified Fund, (ii) 85,993 shares of Common Stock held by Capital Fund, (iii) 84,961 shares of Common Stock held by Opportunity Fund and (iv) 236,246 shares of Common Stock issuable in the aggregate upon the exercise of Pre-Funded Warrants held by Qualified Fund and Capital Fund. Pursuant to the terms of the Pre-Funded Warrants, the Pre-Funded Warrants will be exercisable only to the extent that after giving effect to such exercise the holder thereof and their affiliates would beneficially own no more than 9.99% of outstanding Common Stock of the Company. However, Qualified Fund or Capital Fund may from time to time provide written notice to the Company to increase such beneficial ownership limitation to any other percentage not in excess of 19.99%. Any such change will not be effective until the 61st day after such notice is delivered to the Company. As a result of this restriction, the number of shares of Common Stock that may be issued upon exercise of the Pre-Funded Warrants by Qualified Fund or Capital Fund may change depending upon changes in the outstanding shares of Common Stock. Without giving effect to the above beneficial ownership limitation, the Pre-Funded Warrants that Qualified Fund holds would be exercisable for an aggregate of 1,041,172 shares of Common Stock and the Pre-Funded Warrants that Capital Fund holds would be exercisable for an aggregate of 48,486 shares of Common Stock. EcoR1 is the general partner of Qualified Fund and Capital Fund and the investment advisor to Opportunity Fund. Opportunity GP is the general partner of Opportunity Fund. Oleg Nodelman is the control person of EcoR1 and Opportunity GP and may be deemed to share dispositive and voting power over the shares held by Qualified Fund, Capital Fund and Opportunity Fund. Mr. Nodelman, EcoR1 and Opportunity GP each disclaim beneficial ownership of all shares except to the extent of their pecuniary interest therein. The address of the above persons is 357 Tehama Street #3, San Francisco, CA 94103.
- (7) Shares listed under “Common Stock Beneficially Owned Before the Offering” consist of 1,286,897 shares of Common Stock and 218,964 shares of Common Stock issuable upon the exercise of Pre-Funded Warrants held by the Redmile Funds managed by Redmile, including 652,030 shares of Common Stock held by Redmile Biopharma I. Pursuant to the terms of the Pre-Funded Warrants, the Pre-Funded Warrants will be exercisable only to the extent that after giving effect to such exercise the holder thereof and their affiliates would beneficially own no more than 9.99% of outstanding Common Stock of the Company. However, Redmile may from time to time provide written notice to the Company, to increase such beneficial ownership limitation to any other percentage not in excess of 19.99%. Any such change will not be effective until the 61st day after such notice is delivered to the Company. As a result of this restriction, the number of shares of Common Stock that may be issued upon exercise of the Pre-Funded Warrants by Redmile Funds may change depending upon changes in the outstanding shares of Common Stock. Without giving effect to the above beneficial ownership limitation, the Pre-Funded Warrants that Redmile Funds hold would be exercisable for an aggregate of 263,410 shares of Common Stock, including 160,003 shares of Common Stock issuable upon the exercise of Pre-Funded Warrants held by Redmile Biopharma I. The 500,003 shares listed under “Common Stock that May Be Offered Pursuant to Prospectus” consist of (i) 102,297 shares of Common Stock held directly by Redmile Capital Fund, LP, (ii) 65,211 shares of Common Stock held directly by Redmile Capital Offshore Master Fund, Ltd., (iii) 100,000 shares of Common Stock held directly by Redmile Capital Offshore II Master Fund, Ltd. (iv) 52,452 shares of Common Stock held directly by Redmile Strategic Long Only Trading Sub, Ltd., (v) 20,040 shares of Common Stock held directly by Redmile Strategic Trading Sub, Ltd. and (vi) 160,003 shares of Common Stock issuable upon the exercise of Pre-Funded Warrants held directly by Redmile Biopharma I. Redmile is the investment manager/adviser to the Redmile Funds, and, in such capacity, exercises voting and investment power over all of the shares held by the Redmile Funds and may be deemed to be the beneficial owner of these securities. Jeremy C. Green serves as the managing member of Redmile and also may be deemed to be the beneficial owner of these securities. Redmile and Mr. Green each disclaim beneficial ownership of these securities, except to the extent of its or his pecuniary interest in such securities, if any. The address of the Redmile Funds is c/o Redmile Group, LLC, One Letterman Drive, Building D, Suite D3-300, San Francisco, CA 94129.
- (8) Shares listed under “Common Stock Beneficially Owned Before the Offering” consist of (i) 804,255 shares of Common Stock held by CPM Fund, (ii) 132,217 shares of Common Stock held by CPGE Fund and (iii) 88,144 shares of Common Stock held by CPGE II Fund. CP GP is the general partner of CPM Fund. CPGE GP is the general partner of CPGE Fund, CPGE II GP is the general partner of CPGE II Fund, and Casdin Capital is the investment adviser to each of CPM, CPGE Fund and CPGE II Fund. Eli Casdin is the Managing Member of Casdin Capital, CP GP, CPGE GP and CPGE II GP. Mr. Casdin, Casdin Capital, CP GP, CPGE GP and CPGE II GP disclaim beneficial ownership of any of the reported shares of Common Stock herein, except to the extent of their pecuniary interest therein. The address of the foregoing persons is 1350 Avenue of the Americas, Suite 2600, New York, NY 10019.
- (9) Shares listed under “Common Stock Beneficially Owned Before the Offering” consist of (i) 38,968 shares of Common Stock held by 667, L.P. (“667”) and 414,547 shares of Common Stock held by Baker Brothers Life Sciences, L.P. (“Baker Brothers Life Sciences”) and together with 667, the “Baker Funds”) and (ii) 302,847 shares of Common Stock issuable in the aggregate upon the exercise of Pre-Funded Warrants held by the Baker Funds. Pursuant to the terms of the Pre-Funded Warrants, the Pre-Funded Warrants will be exercisable only to the extent that after giving effect to such exercise the holder thereof and their affiliates would beneficially own no more than 4.99% of the outstanding Common Stock of the Company. However, the Baker Funds may from time to time provide written notice to the Company, to increase such beneficial ownership limitation to any other percentage not in excess of 19.99%. Any such change will not be effective until the 61st day after such notice is delivered to the Company. As a result of this restriction, the number of shares of Common Stock that may be issued upon exercise of the Pre-Funded Warrants by the Baker Funds may change depending upon changes in the outstanding shares of Common Stock. Without giving effect to the above beneficial ownership limitation, the Pre-Funded Warrants that the Baker Funds hold would be exercisable for 360,043 shares of Common Stock by 667 and 4,043,216 shares of Common Stock by Baker Brothers Life Sciences. BBA is the management company and investment advisor to the Baker Funds and has sole voting and investment power with respect to these securities. Baker Bros. Advisors (GP) LLC (the “BBA GP”) is the sole general partner of BBA. Julian C. Baker and Felix J. Baker are managing members of the BBA GP. Julian C. Baker, Felix J. Baker, BBA and the BBA GP may be deemed to be beneficial owners of the securities directly held by the Baker Funds. Julian C. Baker, Felix J. Baker, BBA and the BBA GP disclaim beneficial ownership of all securities held by the Baker Funds, except to the extent of their pecuniary interest therein. The business address of BBA, BBA GP, Julian C. Baker and Felix J. Baker is 860 Washington Street, 3rd Floor, New York, NY 10014.
- (10) Shares listed under “Common Stock Beneficially Owned Before the Offering” consist of 240,000 shares of Common Stock held by Commodore Capital Master LP. Commodore Capital LP is the investment manager to Commodore Capital Master LP and may be deemed to beneficially own the shares held by Commodore Capital Master LP. Michael Kramarz and Robert Egen Atkinson are the managing partners of Commodore Capital LP and exercise investment discretion with respect to these shares. Commodore Capital LP and Commodore Capital Master LP have shared voting and dispositive power with respect to these shares. The address of Commodore Capital LP and Commodore Capital Master LP is 444 Madison Avenue, 35th Floor, New York, NY 10022.

## **Relationship with the Selling Stockholders**

In addition to the SPA, in connection with the PIPE, we entered into the RRA on November 5, 2024 with the Selling Stockholders. Certain of the Investors were, immediately prior to the closing of the PIPE, or became holders of more than 5% of our Common Stock and were parties to the SPA and the RRA. See “*Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters*” and “*Certain Relationships and Related Transactions*.”

## **Registration Rights Agreements**

### *2016 Registration Rights Agreement*

We are party to a Registration Rights Agreement dated September 19, 2016 (the “September 2016 Registration Rights Agreement”) pursuant to which certain of our stockholders have certain registration rights, including the right to demand that we file a registration statement. The registration rights under the September 2016 Registration Rights Agreement will terminate upon the earliest to occur of (a) all of the shares of Neurogene capital stock subject to the demand registration rights under the September 2016 Registration Rights Agreement (the “September 2016 Registrable Securities”) have been sold pursuant to an effective registration statement, (b) the September 2016 Registrable Securities have been sold pursuant to Rule 144 under the Securities Act (or other similar rule); (c) the September 2016 Registrable Securities may be resold without limitations as to volume or manner of sale pursuant to Rule 144 under the Securities Act or (d) 10 years after the date of the September 2016 Registration Rights Agreement.

### *2024 Registration Rights Agreement*

On November 5, 2024, the Company entered into a registration rights agreement with purchasers of shares of Common Stock and pre-funded warrants (the “2024 Registrable Securities”) in the November 2024 Private Placement (the “2024 Registration Rights Agreement”) with the Selling Stockholders named therein pursuant to which, among other things, the Company agreed to file a registration statement covering the resale of the 2024 Registrable Securities by no later than the 30th calendar day following the closing date of the November 2024 Private Placement. See the section titled “Description of Capital Stock—Registration Rights Agreements” located elsewhere in this prospectus for further information on the 2024 Registration Rights Agreement.

## PLAN OF DISTRIBUTION

The Selling Stockholders may, from time to time, sell any or all of their shares of common stock on any stock exchange, market or trading facility on which the shares are traded or in private transactions. These sales may be at fixed or negotiated prices. The Selling Stockholders may use any one or more of the following methods when selling shares:

- ordinary brokerage transactions and transactions in which a broker-dealer solicits purchasers;
- block trades in which a broker-dealer will attempt to sell the shares as agent, but may position and resell a portion of the block as principal to facilitate the transaction;
- purchases by a broker-dealer as principal and resale by the broker-dealer for its account under this prospectus;
- an exchange distribution in accordance with the rules of the applicable exchange;
- privately negotiated transactions;
- “at the market” or through market makers or into an existing market for the shares;
- delivery of securities in settlement of short sales after the date of this prospectus;
- through the writing or settlement of options or other hedging transactions, whether through an options exchange or otherwise;
- through the distribution of the common stock by any Selling Stockholder to its partners, members or stockholders;
- through broker-dealers that agree with any Selling Stockholder to sell a specified number of such shares at a stipulated price per share;
- through one or more underwritten offerings on a firm commitment or best efforts basis;
- a combination of any such methods of sale; and
- any other method permitted pursuant to applicable law.

The Selling Stockholders will act independently of us and each other in making decisions with respect to the timing, manner and size of each sale. The Selling Stockholders also may resell all or a portion of the shares in open market transactions in reliance upon Rule 144, as permitted by that rule, or Section 4(a)(1) under the Securities Act, if available, rather than under this prospectus, provided that they meet the criteria and conform to the requirements of those provisions.

In connection with the sale of the shares, the Selling Stockholders may enter into hedging transactions with broker-dealers or other financial institutions, which may in turn engage in short sales of the common stock in the course of hedging the positions they assume. The Selling Stockholders may also sell shares of our common stock short and deliver these securities to close out its short positions, or loan or pledge the common stock to broker dealers that in turn may sell these securities. The Selling Stockholders may also enter into option or other transactions with broker-dealers or other financial institutions or the creation of one or more derivative securities which require the delivery to such broker-dealer or other financial institution of shares offered by this prospectus, which shares such broker-dealer or other financial institution may resell pursuant to this prospectus (as supplemented or amended to reflect such transaction).

The aggregate proceeds to any Selling Stockholder from the sale of the shares offered by them will be the purchase price of the common stock less discounts or commissions, if any. Each Selling Stockholder reserves the right to accept and, together with its agents from time to time, to reject, in whole or in part, any proposed purchase of common stock to be made directly or through agents. We will not receive any of the proceeds from this offering.

The Selling Stockholders and any broker-dealers that act in connection with the sale of securities may be deemed to be “underwriters” within the meaning of Section 2(a)(11) of the Securities Act in connection with such sales, and any commissions received by such broker-dealers and any profit on the resale of the securities sold by them while acting as principals may be deemed to be underwriting discounts or commissions under the Securities Act. In the event that any Selling Stockholder is deemed to be an “underwriter” within the meaning of Section 2(a)(11) of the Securities Act, such Selling Stockholder will be subject to the prospectus delivery requirements of the Securities Act. A Selling Stockholder may indemnify any broker-dealer that participates in transactions involving the sale of the shares against certain liabilities, including liabilities arising under the Securities Act. To our knowledge, none of the Selling Stockholders have received any shares as underwriting compensation.

A Selling Stockholder may from time to time pledge or grant a security interest in some or all of the shares of common stock beneficially owned by it and, if it defaults in the performance of its secured obligations, the pledgees or secured parties may offer and sell the shares of common stock from time to time under this prospectus after we have filed a supplement to this prospectus, or, if required, a post-effective amendment to the registration statement of which this prospectus is a part, supplementing or amending the list of Selling Stockholders to include the pledgee, transferee or other successors in interest as Selling Stockholders under this prospectus.

The Selling Stockholders also may transfer the shares of common stock in other circumstances, in which case the transferees or other successors in interest will be the selling beneficial owners for purposes of this prospectus and may sell the shares of common stock from time to time under this prospectus after we have filed a supplement to this prospectus or, if required, a post-effective amendment to the registration statement of which this prospectus is a part, supplementing or amending the list of Selling Stockholders to include the transferee or other successors in interest as Selling Stockholders under this prospectus.

To the extent required, the shares of our common stock to be sold, the names of the Selling Stockholders, the respective purchase prices and public offering prices, the names of any agents, dealer or underwriter, any applicable commissions or discounts with respect to a particular offer will be set forth in an amendment or supplement to this prospectus.

We will pay all registration expenses incurred relating to the registration of the shares with the SEC, subject to reimbursement by the Selling Stockholders, pursuant to the Registration Rights Agreement, and the Selling Stockholders will pay all selling expenses, including underwriting discounts and selling commissions, related to the sale of the shares registered under this registration statement. We have agreed to indemnify the Selling Stockholders against certain losses, claims, damages and liabilities, including liabilities under the Securities Act.

Each of the Selling Stockholders has advised us that it has not entered into any agreements, understandings or arrangements with any underwriters or broker-dealers regarding the sale of their shares of common stock, nor is there an underwriter or coordinating broker acting in connection with a proposed sale of shares of common stock by the Selling Stockholder. If we are notified by a Selling Stockholder that any material arrangement has been entered into with a broker-dealer for the sale of shares of common stock, if required, we will file a supplement to this prospectus. If a Selling Stockholder uses this prospectus for any sale of the shares of common stock, it will be subject to the prospectus delivery requirements of the Securities Act.

We have advised the Selling Stockholders that the anti-manipulation rules of Regulation M under the Exchange Act may apply to sales of shares of our common stock in the market and to the activities of the Selling Stockholders and their respective affiliates. These rules may limit the timing of purchases and sales of the shares by any Selling Stockholder. Regulation M may also restrict the ability of any person engaged in the distribution of the shares of common stock to engage in market-making activities with respect to the shares of common stock. All of the foregoing may affect the marketability of the shares of common stock and the ability of any person or entity to engage in market-making activities with respect to the shares of common stock.

Our common stock is listed on The Nasdaq Global Market under the symbol “NGNE.” There can be no assurance that any Selling Stockholder will sell any or all of the shares of common stock registered pursuant to this prospectus.



## DESCRIPTION OF CAPITAL STOCK

The following description of our capital stock is intended as a summary only and therefore is not a complete description of our capital stock. This description is based upon, and is qualified by reference to, our certificate of incorporation (“charter”), our bylaws (“bylaws”) and applicable provisions of Delaware corporate law. You should read our charter and our bylaws, copies of which are filed as exhibits to the registration statement of which this prospectus forms a part.

### **Authorized Capital Stock**

Our authorized capital stock consists of 450,000,000 shares of common stock, par value \$0.000001 per share (“Common Stock”) and 50,000,000 shares of preferred stock, par value \$0.000001 per share (“Preferred Stock”).

### ***Common Stock***

Our charter authorizes the issuance of up to 450,000,000 shares of our common stock. All outstanding shares of our common stock are validly issued, fully paid and nonassessable.

### ***Voting***

The holders of Common Stock are entitled to one vote per share on all matters submitted to a vote of stockholders, except for matters relating solely to the terms of Preferred Stock on which holders of our Common Stock shall not be entitled to vote as provided in our charter. A majority vote of the shares present in person or represented by proxy and entitled to vote on the subject matter is required for the holders of our Common Stock to take action on all matters, except as otherwise required by law, our charter or our bylaws. Our charter does not provide for cumulative voting in the election of directors.

### ***Dividends***

Subject to preferences that may be applicable to any then-outstanding preferred stock, holders of common stock are entitled to receive ratably those dividends, if any, as may be declared from time to time by the board of directors out of legally available funds.

### ***Distributions on Liquidation***

In the event of our liquidation, dissolution or winding up, holders of Common Stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then-outstanding shares Preferred Stock.

### ***Rights and Preferences***

Holders of Common Stock have no preemptive, conversion or subscription rights and there are no redemption or sinking fund provisions applicable to our Common Stock. The rights, preferences and privileges of the holders of common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of preferred stock that we may designate in the future.

### **Preferred Stock**

Under the terms of our charter, our board of directors has the authority, without further action by our stockholders, to issue up to 50,000,000 shares of Preferred Stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the dividend, voting and other rights, preferences and privileges of the shares of each wholly unissued series and any qualifications, limitations or restrictions thereon, and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding. Our board of directors may authorize the issuance of Preferred Stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of our Common Stock. The issuance of Preferred Stock, while providing flexibility in connection with possible acquisitions and other

corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in our control and may adversely affect the market price of our common stock and the voting and other rights of the holders of our Common Stock.

#### **Anti-Takeover Effects of Delaware Law and Provisions of Our Charter and Bylaws**

Our charter and bylaws include a number of provisions that may have the effect of delaying, deferring or preventing another party from acquiring control of us and encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts.

- **Issuance of undesignated Preferred Stock:** Under our charter, our board of directors has the authority, without further action by the stockholders, to issue up to 50,000,000 shares of undesignated Preferred Stock with rights and preferences, including voting rights, designated from time to time by our board of directors. The existence of authorized but unissued shares of Preferred Stock enables our board of directors to make it more difficult to attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise.
- **Classified board:** Our charter establishes a classified board of directors consisting of three classes of directors, with staggered three-year terms. Only one class of directors is elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. This provision may have the effect of delaying a change of control of our board of directors.
- **Election and removal of directors and board vacancies:** Our bylaws provide that directors will be elected by a plurality vote. Our charter and bylaws also provide that our board of directors has the right to increase or decrease the size of the board to fill vacancies on the board. Directors may be removed only for cause by the affirmative vote of the holders of at least 66 2/3% of the votes that all our stockholders would be entitled to cast in an annual election of directors. Only our board of directors is authorized to fill vacant directorships. In addition, the number of directors constituting our board of directors may be set only by resolution adopted by a majority of the whole board of directors. These provisions prevent stockholders from increasing the size of our board of directors and gaining control of our board of directors by filling the resulting vacancies with its own nominees.
- **Requirements for advance notification of stockholder nominations and proposals:** Our bylaws establish advance notice procedures with respect to stockholder proposals and the nomination of candidates for election as directors that specify certain requirements as to the timing, form and content of a stockholder's notice. Business that may be conducted at an annual meeting of stockholders will be limited to those matters properly brought before the meeting. These provisions may make it more difficult for our stockholders to bring matters before our annual meeting of stockholders or to nominate directors at annual meetings of stockholders.
- **No written consent of stockholders:** Our charter provides that all stockholder actions be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting. This limit may lengthen the amount of time required to take stockholder actions and would prevent the amendment of our bylaws or removal of directors by our stockholders without holding a meeting of stockholders.
- **No stockholder ability to call special meetings:** Our bylaws provide that only a majority of the members of our board of directors then in office may be able to call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders.
- **Amendments to certificate of incorporation and bylaws:** Any amendment to our charter will be required to be approved by a majority of our board of directors as well as, if required by law or the charter, a majority of the outstanding shares entitled to vote on the amendment and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of provisions to board

classification, election, removal and vacancies, stockholder action, certificate amendments, bylaw amendments, liability of directors, indemnification of directors and officers, and Delaware exclusive forum must be approved by not less than 66 2/3% of the outstanding shares entitled to vote on the amendment, voting together as a single class. Any amendment to our bylaws must be approved by either a majority of the authorized number of directors constituting the board of directors or not less than 66 2/3% of the outstanding shares entitled to vote on the amendment, voting together as a single class.

These provisions are designed to enhance the likelihood of continued stability in the composition of our board of directors and its policies, to discourage certain types of transactions that may involve an actual or threatened acquisition of our company and to reduce our vulnerability to an unsolicited acquisition proposal. We also designed these provisions to discourage certain tactics that may be used in proxy fights. However, these provisions could have the effect of discouraging others from making tender offers for our shares and, as a consequence, they may also reduce fluctuations in the market price of our shares that could result from actual or rumored takeover attempts.

### ***Section 203 of the Delaware General Corporation Law***

We are subject to the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a “business combination” with an “interested stockholder” for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in advance by a majority of the independent directors or by the holders of at least two-thirds of the outstanding disinterested shares. The application of Section 203 of the Delaware General Corporation Law could also have the effect of delaying or preventing a change of control of us.

### ***Choice of Forum***

Our charter requires that the Court of Chancery of the State of Delaware be the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (1) any derivative action or proceeding brought on our behalf; (2) any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee to us or our stockholders; (3) any action asserting a claim against us or any director or officer or other employee arising pursuant to the Delaware General Corporation Law or our charter or bylaws; or (4) any action asserting a claim against us that is governed by the internal affairs doctrine. This provision would not apply to claims brought to enforce a duty or liability created by the Securities Exchange Act of 1934, as amended, or any other claim for which the federal courts have exclusive jurisdiction. Our bylaws provide further that the federal district courts of the United States will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act of 1933, as amended. The forum selection provisions will not apply to claims brought to enforce a duty or liability created by the Securities Exchange Act of 1934, as amended. It is possible that a court could find that such provisions are inapplicable for a particular claim or action or that such provisions are unenforceable. In addition, under the Securities Act, federal courts have concurrent jurisdiction over all suits brought to enforce any duty or liability created by the Securities Act, and investors cannot waive compliance with the federal securities laws and the rules and regulations thereunder. Although we believe these provisions benefit us by providing increased consistency in the application of Delaware law for the specified types of actions and proceedings, the provisions may have the effect of discouraging lawsuits against us or our directors or officers.

### ***Registration Rights Agreements***

#### ***2016 Registration Rights Agreement***

In connection with our public offering of common stock on September 19, 2016, we entered into the 2016 Registration Rights Agreement with certain holders of our securities. Under the 2016 Registration Rights Agreement, we agree that, if at any time and from time to time after December 19, 2016, those investors demand that we register their shares of our Common Stock for resale under the Securities Act, we would be obligated to effect such registration. We have registered an aggregate of approximately 1,292,268 shares (after giving effect to the 1-for-5 reverse stock split in September 2023 and the subsequent 1-for-4 reverse stock split in December 2023) for resale, from time to time, by such stockholders. Our registration obligations under the 2016 Registration Rights Agreement covers all shares now held or hereafter acquired by the stockholders who are a party to such agreement

and is in effect until the earlier of (a) the date when all of the 2016 Registrable Securities subject to the demand registration rights under the 2016 Registration Rights Agreement have been sold pursuant to an effective registration statement, (b) the 2016 Registrable Securities have been sold pursuant to Rule 144 under the Securities Act (or other similar rule); (c) the 2016 Registrable Securities may be resold without limitations as to volume or manner of sale pursuant to Rule 144 under the Securities Act or (d) 10 years after the date of the 2016 Registration Rights Agreement, and includes our obligation to facilitate certain underwritten public offerings of our common stock by these stockholders in the future.

The foregoing description of the 2016 Registration Rights Agreement does not purport to be complete and is qualified in its entirety by the full text of the 2016 Registration Rights Agreement, a copy of which is filed as Exhibit 4.2 to this Registration Statement.

#### **2024 Registration Rights Agreement**

In connection with the closing of the November 2024 Private Placement, on November 5, 2024, we entered into the 2024 Registration Rights Agreement with the Selling Shareholders, pursuant to which, among other things, we agreed to file a registration statement covering the resale of the 2024 Registrable Securities by no later than the 30th calendar day following the closing date of the November 2024 Private Placement (the “**Filing Deadline**”). In addition, we have agreed to use our best efforts to cause such registration statement to become effective as soon as practicable, but in any event no later than the earlier of (a) the 105th calendar day following the Closing Date or, (b) the fifth business day after the date we are notified (orally or in writing, whichever is earlier) by the Securities and Exchange Commission that the registration statement will not be “reviewed” or will not be subject to further review (the “**Effectiveness Date**”). We are required to use our best efforts to keep such registration statement continuously effective until the date that all 2024 Registrable Securities covered by such registration statement (i) have been sold, thereunder or pursuant to Rule 144, or (ii) may be sold without volume or manner-of-sale restrictions pursuant to Rule 144 and without the requirement for us to be in compliance with the current public information requirement under Rule 144. We will be responsible for all fees and expenses incurred in connection with the registration of the Registrable Securities.

The Registration Rights Agreement requires us to provide certain customary indemnification to the Selling Shareholders in connection with the registration statement.

The foregoing description of the Registration Rights Agreement does not purport to be complete and is qualified in its entirety by the full text of the 2024 Registration Rights Agreement, a copy of which is filed as Exhibit 4.3 to this Registration Statement.

#### **Transfer Agent and Registrar**

Equiniti Trust Company, LLC serves as the transfer agent and registrar for our Common Stock.

#### **Listing**

Our Common Stock is listed on the Nasdaq Global Market under the symbol “NGNE.”

## LEGAL MATTERS

Certain legal matters, including the legality of the securities offered, has been passed upon for us by Gibson, Dunn & Crutcher LLP, San Francisco, California. Additional legal matters may be passed upon for us or any underwriters, dealers or agents, by counsel that we will name in the applicable prospectus supplement.

## EXPERTS

The financial statements of Neurogene Inc. as of December 31, 2023 and for the period ended December 31, 2023, included in this prospectus, have been audited by Deloitte & Touche LLP, an independent registered public accounting firm, as stated in their report. Such financial statements are included in reliance upon the report of such firm given their authority as experts in accounting and auditing.

The financial statements of Neurogene at December 31, 2022 and for the year then ended, appearing in this Prospectus and Registration Statement have been audited by Ernst & Young LLP, independent registered public accounting firm, as set forth in their report thereon appearing elsewhere herein, and are included in reliance upon such report given on the authority of such firm as experts in accounting and auditing.

## WHERE YOU CAN FIND MORE INFORMATION

We are subject to the informational requirements of the Exchange Act and are required to file annual, quarterly and other reports, proxy statements and other information with the SEC. The SEC maintains an Internet site (<http://www.sec.gov>) that contains reports, proxy and information statements, and various other information about us.

Information about us is also available at our website at <http://www.neurogene.com>. You may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. However, the information on our website is not a part of this prospectus and is not incorporated by reference into this prospectus.

We have filed a registration statement on Form S-1 with the SEC relating to the securities covered by this prospectus. This prospectus is a part of the registration statement and does not contain all of the information in the registration statement. Whenever a reference is made in this prospectus to a contract or other document of ours, please be aware that the reference is only a summary and that you should refer to the exhibits that are part of the registration statement for a copy of the contract or other document. You may review a copy of the registration statement through the SEC's website or our website.

**NEUROGENE INC.**

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## Report of Independent Registered Public Accounting Firm

To the Shareholders and the Board of Directors of Neurogene Inc.

### Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Neurogene Inc. and subsidiaries (the "Company") as of December 31, 2023, the related consolidated statements of operations, changes in convertible preferred stock and stockholders' equity (deficit), and cash flows for the year ended December 31, 2023, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2023, and the results of its operations and its cash flows for the year ended December 31, 2023, in conformity with accounting principles generally accepted in the United States of America.

### Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audit, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audit included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audit provides a reasonable basis for our opinion.

### Critical Audit Matters

The critical audit matters communicated below are matters arising from the current-period audit of the financial statements that were communicated or required to be communicated to the audit committee and that (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the financial statements, taken as a whole, and we are not, by communicating the critical audit matters below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

#### ***Reverse Merger Acquisition — Accounting Conclusions — Refer to Note 1, 3, 4 and 10 to the financial statements.***

##### *Critical Audit Matter Description*

The reverse merger between Neoleukin Therapeutics, Inc. ("Neoleukin"), one of Neoleukin's wholly owned subsidiaries, and Neurogene Inc. ("Neurogene") was accounted for as a reverse asset acquisition pursuant to ASC 805, *Business Combinations*, as Neoleukin did not meet the definition of a business under ASC 805 but it did represent a group of assets. The initial accounting conclusions related to the reverse merger involved determining: (1) the accounting acquirer and accounting acquiree; (2) whether the transaction should be accounted for as a business combination or an asset acquisition; and (3) whether contingent consideration liabilities associated with the

Contingent Value Rights (CVRs) should be accounted for as a derivative at fair value or recognized when payments are probable and estimable as defined by ASC 450, *Contingencies*.

Given the significant judgment required by management in concluding that Neurogene is the accounting acquirer in the reverse merger transaction, the transaction be accounted for as a reverse asset acquisition, and the accounting for the contingent consideration liabilities associated with the CVRs, the audit procedures performed to evaluate the reasonableness of management's conclusions related to these accounting conclusions required a high degree of auditor judgment, including the need to involve professionals in our firm with the appropriate expertise in accounting for business combinations and derivatives.

*How the Critical Audit Matter Was Addressed in the Audit*

Our audit procedures related to evaluating the reasonableness of management's accounting conclusions related to the determination of the accounting acquirer and accounting for the transaction as a reverse asset acquisition and accounting for contingent consideration liabilities associated with the CVRs included the following, among others:

- We obtained and read the Agreement and Plan of Merger (the "Merger Agreement").
- With the assistance of professionals in our firm with expertise in accounting for business combinations, we evaluated the Company's accounting conclusions for the reverse merger, including determining whether Neurogene should be the accounting acquirer and whether the transaction should be accounted for as a reverse asset acquisition based on the terms of the Merger Agreement.
- With the assistance of professionals in our firm with expertise in accounting for business combinations and derivatives, we evaluated the Company's accounting conclusions for contingent consideration liabilities associated with the CVRs.

***Accrued research and development expenses— Refer to Note 3 and Note 9 to the financial statements***

*Critical Audit Matter Description*

The Company incurs certain research and development expenses from third-party contract research organizations. The Company accrues expenses for preclinical studies and clinical trial activities performed by its vendors based upon estimates of the proportion of work completed. The Company determines estimates of the work completed under the contracts based on review of the contracts, vendor agreements and purchase orders, and through discussions with internal clinical personnel and external service providers to determine stage of completion of trials or services and the agreed-upon fee to be paid for such services.

Given the judgement required by management to estimate the extent of services performed and the associated expenses incurred as it relates to research and development costs incurred, performing audit procedures to evaluate accrued research and development expenses required a high degree of auditor judgement and an increased extent of effort.

*How the Critical Audit Matter Was Addressed in the Audit*

Our audit procedures related to accrued research and development expenses included the following, among others:

- We evaluated publicly available information (such as press releases and investor presentations) and board of directors' materials regarding the status of research and development activities.
- We selected a sample of vendors and tested the related accrual of research and development expenses by:
  - Confirming the completeness of the terms and conditions of research and development service agreements directly with the vendor.
  - Obtaining and reading the related contracts to understand key provisions and agree them to the Company's analysis.



- Obtaining and inspecting third-party documents such as status reports and other correspondence received from the vendors related to the services provided and comparing them to the Company's schedule of estimated expenses incurred to date.
- Inspecting meeting minutes between the Company's finance team and clinical and manufacturing operations and corroborating the progress of research and development activities through inquiry with the Company's clinical operations personnel.
- Examining invoices received from vendors and cash disbursements, where applicable, made subsequent to the balance sheet date and inquiring of clinical operations to corroborate the applicable service period in order to evaluate completeness of the accruals.

/s/ Deloitte & Touche LLP

Seattle, WA  
March 18, 2024

We have served as the Company's auditor since 2023.

## **Report of Independent Registered Public Accounting Firm**

To the Stockholders and the Board of Directors of Neurogene Inc.

### **Opinion on the Financial Statements**

We have audited the accompanying balance sheet of Neurogene Inc. (the Company) as of December 31, 2022, the related statements of operations, changes in convertible preferred stock and stockholders' deficit and cash flows for the year ended December 31, 2022, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2022, and the results of its operations and its cash flows for the year then ended, in conformity with U.S. generally accepted accounting principles.

### **Basis for Opinion**

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audit we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audit included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audit provides a reasonable basis for our opinion.

### **Critical Audit Matters**

The critical audit matters communicated below are matters arising from the current period audit of the financial statements that were communicated or required to be communicated to the audit committee and that: (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the financial statements, taken as a whole, and we are not, by communicating the critical audit matters below, providing separate opinions on the critical audit matters or on the accounts or disclosures to which they relate.

### **Accrued research and development expenses**

*Description of the Matter*

As more fully described in Note 3 to the financial statements, the Company records accrued liabilities for estimated costs of research and development (“R&D”) activities based upon the estimated cost of services provided but not yet invoiced. As of December 31, 2022, accrued clinical costs and other R&D costs were \$1.8 million. The accrued costs included estimated clinical trial and other R&D costs incurred but not invoiced under certain significant R&D service agreements with organizations that conduct R&D activities on behalf of the Company (“Accrued R&D Costs”). The Company accrues for these costs based on several factors, such as information obtained from vendors and estimates of the work completed under the service agreements. Auditing the Company's accounting for Accrued R&D Costs was complex because the Company's analysis is dependent upon data obtained from external third-party service providers who often act as intermediaries to those performing the underlying services. The determination of the accrual when the Company has either not been invoiced or has not received information regarding actual costs incurred requires evaluation of the stage of completion of the services.

*How We Addressed the Matter in Our Audit*

To test the Accrued R&D Costs, our audit procedures included, among others, i) confirming the completeness of the terms and conditions of certain significant R&D service agreements directly with the vendor; ii) testing the completeness and accuracy of the Company's accrual models through verification of significant inputs, such as costs incurred and invoices paid, to the terms and conditions of the underlying agreements and information from the Company's internal personnel and vendors; iii) meeting with personnel outside of the accounting department to discuss the basis for assumptions used in estimating cost of services provided but not yet invoiced; and iv) performing a hindsight analysis of invoices received subsequent to the balance sheet date.

### **Valuation of common stock**

*Description of the Matter*

As discussed in Note 3 and Note 14 to the financial statements, the Company accounts for stock options granted to employees and non-employees at fair value using Black-Scholes Option pricing model. The valuation of common stock, among other inputs, was used to determine the fair value measurement for stock-based awards granted. The Company's common stock was not publicly traded on the dates which the stock-based awards were granted and required management to estimate its value with the assistance of an unrelated third-party valuation firm using methodologies, approaches and assumptions consistent with the American Institute of Certified Public Accountants, Audit and Accounting Practice Aid Series: Valuation of Privately Held Company Equity Securities Issued as Compensation. Auditing the Company's valuation of common stock was complex and required auditor's judgment due to significant assumptions which included, among others, discount for lack of marketability and probability weighting the likelihood of achieving a liquidity event for the holders of the Company's common stock. The Company recognized \$1.3 million in stock-based compensation expense for the year ending December 31, 2022.

*How We Addressed the Matter in Our Audit*

To test the valuation of common stock, our audit procedures included, among others, assessing the competence of the third-party valuation firm and the valuation methodologies by considering the Company's equity structure, the proximity of sales of convertible preferred stock to investors in arm's length transactions, and possible exit scenarios used in estimating the fair value of the common stock. We also evaluated whether the calculations supporting the estimate were applied in accordance with acceptable approaches and mathematically accurate, evaluated the reasonableness of the discount for lack of marketability used in the valuation and performed sensitivity analyses of the significant assumptions to evaluate the change in the fair value resulting from changes in the assumptions. Internal valuation specialists assisted us in performing these procedures. In addition, we assessed the appropriateness of comparable companies used to determine multiples used in market approach estimates applied to Company financial data.

/s/ Ernst & Young LLP

We served as the Company's auditor from 2019 to 2023.

Stamford, Connecticut

August 18, 2023, except for the effects of the exchange ratio disclosed in Note 1, sub-section titled Reverse Merger and Pre-Closing Financing, as to which the date is March 18, 2024.

**Neurogene Inc.**  
**Consolidated Balance Sheets**  
(In Thousands, Except Share Information)

	December 31, 2023	December 31, 2022
<b>Assets</b>		
Current Assets:		
Cash and cash equivalents	\$ 148,210	\$ 82,021
Short-term investments	48,947	—
Prepaid expenses and other current assets	3,191	2,698
Total current assets	200,348	84,719
Property and equipment, net	17,174	20,115
Operating lease right-of-use assets	3,681	4,344
Finance lease right-of-use assets	98	87
Restricted cash	508	—
Other non-current assets	764	—
Total assets	\$ 222,573	\$ 109,265
<b>Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)</b>		
Current liabilities:		
Accounts payable	\$ 2,596	\$ 625
Accrued expenses and other current liabilities	17,495	5,324
Operating lease liabilities, current	2,559	678
Finance lease liabilities, current	42	24
Lease contingent value rights liability, current	281	—
Total current liabilities	22,973	6,651
Operating lease liabilities, non-current	12,302	3,921
Finance lease liabilities, non-current	65	66
Lease contingent value rights liability, non-current	1,006	—
Other liabilities	203	—
Total liabilities	36,549	10,638
Commitments and contingencies (Note 10)		
Convertible preferred stock:		
Series A-1 Convertible Preferred stock, \$0.0001 par value; 0 and 18,604,653 shares authorized, issued and outstanding as of December 31, 2023 and 2022, respectively (liquidation value of \$40,000 at December 31, 2022)	—	34,414
Series A-2 Convertible Preferred stock, \$0.0001 par value; 0 and 13,291,208 shares authorized, issued and outstanding as of December 31, 2023 and 2022, respectively (liquidation value of \$28,675 at December 31, 2022)	—	28,675
Series B Convertible Preferred stock, \$0.0001 par value; 0 and 88,114,739 shares authorized, 0 and 74,405,719 shares issued and outstanding as of December 31, 2023 and 2022, respectively (liquidation value of \$181,550 at December 31, 2022)	—	181,277
Total convertible preferred stock	—	244,366
Stockholders' equity (deficit)		
Preferred stock, \$0.000001 par value; 50,000,000 and 0 shares authorized as of December 31, 2023 and 2022, respectively; 0 shares issued and outstanding as of December 31, 2023 and 2022, respectively	—	—
Class A Common stock, \$0.0001 par value; 0 and 126,000,000 shares authorized as of December 31, 2023 and 2022, respectively; 0 and 428,334 shares issued and outstanding as of December 31, 2023 and 2022, respectively	—	—
Class B Common stock, \$0.0001 par value; 0 and 120,010,600 shares authorized as of December 31, 2023 and 2022, respectively; 0 shares issued and outstanding as of December 31, 2023 and 2022, respectively	—	—
Common stock, \$0.000001 par value; 450,000,000 and 0 shares authorized as of December 31, 2023 and 2022, respectively; 12,823,665 and 0 shares issued and outstanding as of December 31, 2023 and 2022, respectively	—	—
Additional paid-in capital	373,178	5,098
Accumulated deficit	(187,154)	(150,837)
Total stockholders' equity (deficit)	186,024	(145,739)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	\$ 222,573	\$ 109,265

*The accompanying notes are an integral part of these consolidated financial statements.*

**Neurogene Inc.**  
**Consolidated Statements of Operations**  
(In Thousands, Except Share Information)

	Year Ended December 31,	
	2023	2022
Operating expenses:		
Research and development	\$ 44,394	\$ 47,505
General and administrative	11,189	9,012
Total operating expenses	55,583	56,517
Loss from operations	(55,583)	(56,517)
Other income (expense):		
Interest income	2,951	1,337
Interest expense	(12)	(2)
Bargain purchase gain	16,355	—
Other expense	(28)	(7)
Net loss	\$ (36,317)	\$ (55,189)

	Pre-Merger		Post-Merger	Pre-Merger	
	(a)	(b)	(c)	(a)	(b)
Per share information <sup>(1)</sup> :					
Net income (loss) per share, basic	\$ (117.28)	\$ —	\$ 27.76	\$ (139.88)	\$ —
Weighted-average shares outstanding used in computing net income (loss) per share, basic	426,097	—	491,867	394,533	—
Net income (loss) per share, diluted	\$ (117.28)	\$ —	\$ 2.93	\$ (139.88)	\$ —
Weighted-average shares outstanding used in computing net income (loss) per share, diluted	426,097	—	4,656,947	394,533	—

(1) On December 18, 2023, the Company completed its reverse merger, which among other things, resulted in Neurogene OpCo merging with and into a wholly owned subsidiary of Neoleukin Therapeutics, Inc. As the earnings per share information for the pre-merger period is not comparable to the earnings per share information for the post-merger period, the earnings per share information is being presented separately for these periods. See Note 3, *Net Income (Loss) Per share*, for additional information.

- (a) Presents information for the pre-merger period for Class A common stock. The pre-merger period is January 1, 2023 through December 17, 2023 for the year ended December 31, 2023 and the full fiscal year ended December 31, 2022.
- (b) Presents information for the pre-merger period for Class B common stock. The pre-merger period is January 1, 2023 through December 17, 2023 for the year ended December 31, 2023 and the full fiscal year ended December 31, 2022.
- (c) Presents information for the post-merger period for common stock. The post-merger period is December 18, 2023 through December 31, 2023.

*The accompanying notes are an integral part of these consolidated financial statements.*

**Neurogene Inc.**  
**Consolidated Statements of Changes in Convertible Preferred Stock and Stockholders' Equity (Deficit)**  
(In Thousands, Except Share Information)

	Convertible Preferred Stock						Stockholders' equity (deficit)										
	Series A-1 Convertible Preferred Stock		Series A-2 Convertible Preferred Stock		Series B Convertible Preferred Stock		Preferred Stock		Class A Common Stock		Class B Common Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount			
<b>Balance- December 31, 2021</b>	18,604,653	\$ 34,414	13,291,208	\$ 28,675	47,131,133	\$ 114,818	—	\$ —	419,783	\$ —	—	\$ —	—	\$ —	\$ 3,773	\$ (95,648)	\$ (91,875)
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	—	—	1,252	—	1,252
Series B convertible preferred stock, net of \$91 offering costs	—	—	—	—	27,274,586	66,459	—	—	—	—	—	—	—	—	—	—	—
Class A common stock issued upon exercise of stock options	—	—	—	—	—	—	—	—	8,551	—	—	—	—	—	73	—	73
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(55,189)	(55,189)
<b>Balance- December 31, 2022</b>	18,604,653	\$ 34,414	13,291,208	\$ 28,675	74,405,719	\$ 181,277	—	\$ —	428,334	\$ —	—	\$ —	—	\$ —	\$ 5,098	\$ (150,837)	\$ (145,739)
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	—	—	1,418	—	1,418
Class A common stock issued upon exercise of stock options	—	—	—	—	—	—	—	—	20,348	—	—	—	—	—	168	—	168
Additional convertible preferred stock issued in conjunction with anti-dilution adjustment	1,890,302	—	1,350,436	—	10,264,533	—	—	—	—	—	—	—	—	—	—	—	—
Conversion of convertible preferred stock into common stock and pre-funded warrants in connection with the reverse merger	(20,494,955)	(34,414)	(14,641,644)	(28,675)	(84,670,252)	(181,277)	—	—	—	—	—	—	7,231,747	—	244,366	—	244,366
Issuance of Class A common stock and pre-funded warrants in the pre-closing financing, net of \$6,946 offering costs	—	—	—	—	—	—	—	—	2,792,206	—	—	—	—	—	88,056	—	88,056
Conversion of Class A common stock into common stock and related change in par value	—	—	—	—	—	—	—	—	(3,240,888)	—	—	—	3,240,888	—	—	—	—
Transactions costs associated with the reverse merger	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(4,140)	—	(4,140)
Issuance of common stock and pre-funded warrants to former stockholders of Neoleukin Therapeutics, Inc. in connection with the reverse merger	—	—	—	—	—	—	—	—	—	—	—	—	2,351,030	—	38,212	—	38,212
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(36,317)	(36,317)
<b>Balance- December 31, 2023</b>	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	12,823,665	\$ —	\$ 373,178	\$ (187,154)	\$ 186,024

*The accompanying notes are an integral part of these consolidated financial statements.*

**Neurogene Inc.**  
**Consolidated Statements of Cash Flows**  
(In Thousands)

	Year Ended December 31,	
	2023	2022
<b>Cash flows used in operating activities:</b>		
Net loss	\$ (36,317)	\$ (55,189)
<b>Adjustments to reconcile net loss to net cash used in operating activities:</b>		
Stock-based compensation expense	1,418	1,252
Bargain purchase gain in connection with reverse merger	(16,355)	—
Depreciation and amortization of property and equipment	3,262	3,196
Non-cash operating lease expense	663	608
Amortization of finance lease right-of-use assets	35	4
Amortization and accretion of premiums/discounts on held-to-maturity investments	(105)	—
<b>Changes in assets and liabilities:</b>		
Prepaid expenses and other current assets	326	716
Other assets	(764)	—
Accounts payable	667	(3,607)
Accrued expenses and other current liabilities	(3,574)	806
Operating lease liabilities	(678)	(610)
Net cash used in operating activities	(51,422)	(52,824)
<b>Cash flows used in investing activities:</b>		
Purchases of property and equipment	(321)	(2,230)
Cash and restricted cash acquired from reverse merger transaction	22,243	—
Reverse merger transaction costs paid	(1,285)	—
Proceeds from maturities of held-to-maturity investments	5,000	—
Net cash used in investing activities	25,637	(2,230)
<b>Cash flows provided by financing activities:</b>		
Proceeds from pre-closing financing, net of offering costs paid	92,343	—
Proceeds from issuance of Series B convertible preferred stock, net of offering costs paid	—	66,459
Proceeds from the issuance of common stock upon exercise of options	168	73
Principal payments on finance leases	(29)	(1)
Net cash provided by financing activities	92,482	66,531
Net increase in cash, cash equivalents and restricted cash	66,697	11,477
Cash, cash equivalents and restricted cash at beginning of year	82,021	70,544
Cash, cash equivalents and restricted cash at end of year	\$ 148,718	\$ 82,021
<b>Supplemental disclosure of non-cash investing and financing activities:</b>		
Additions to finance lease right of use assets from new finance lease liabilities	\$ 46	\$ 91
Operating lease liabilities assumed in connection with the reverse merger	\$ 10,940	\$ —
Contingent consideration liability assumed in the reverse merger	\$ 1,287	\$ —
Other liabilities assumed in the reverse merger	\$ 10,110	\$ —
Assets acquired in the reverse merger including \$53.9 million in short-term investments and \$0.8 million in other monetary assets	\$ 54,661	\$ —
Issuance of common stock and pre-funded warrants to former stockholders of Neoleukin Therapeutics, Inc. in connection with the reverse merger	\$ 38,212	\$ —
Offering costs in connection with pre-closing financing included in accounts payable and accrued expenses	\$ 4,287	\$ —
Transaction costs related to reverse merger included in accounts payable and accrued expenses	\$ 2,855	\$ —
Conversion of preferred stock into common stock in connection with reverse merger	\$ 244,366	\$ —
<b>Supplemental cash flow information:</b>		
Cash paid for interest	\$ 12	\$ 2

*The accompanying notes are an integral part of these consolidated financial statements.*



## NEUROGENE INC.

### NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

#### 1. Organization and Description of Business

Neurogene Inc. (formerly known as Neoleukin Therapeutics, Inc.) (the “Company” or “Neurogene”) is a clinical stage biotechnology company that is a result of the reverse merger discussed below. Pre-merger Neurogene Inc. was incorporated as a limited liability company in Delaware on January 26, 2018 and converted into a corporation on July 3, 2018, and has a principal place of business in New York, NY. The Company was formed to harness the power of gene therapy, combined with its EXACT gene regulation technology, to turn today’s complex devastating neurological diseases into treatable conditions. The Company’s first clinical-stage program to utilize the EXACT technology is NGN-401, which is under development for the treatment of Rett syndrome. Since beginning operations, the Company has devoted substantially all its efforts to research and development, recruiting management and technical staff, administration, and raising capital.

#### *Reverse Merger and Pre-Closing Financing*

On July 18, 2023, Neoleukin Therapeutics, Inc. entered into an Agreement and Plan of Merger (the “Merger Agreement”) with one of its wholly owned subsidiaries (“Merger Sub”) and Neurogene Inc. (“Neurogene OpCo”). Pursuant to the terms of the Merger Agreement, upon closing on December 18, 2023 (the “Closing”), Merger Sub merged with and into Neurogene OpCo, with Neurogene OpCo continuing as a wholly owned subsidiary of the Company and the surviving corporation of the merger (referred to herein as the “reverse merger”).

At the time of Closing (or immediately prior to, where indicated), the following also occurred:

- The Company changed its name from “Neoleukin Therapeutics, Inc.” to “Neurogene Inc.” and is referred to herein as the “Company.” Unless the context otherwise requires, references to “Neoleukin Therapeutics, Inc.” or “Neoleukin” refer to the Company prior to Closing.
- Immediately prior to Closing, Neoleukin effected a 1-for-4 reverse stock split (the “Reverse Stock Split”). Unless noted otherwise, all references herein to share and per share amounts reflect the Reverse Stock Split.
- All of the then outstanding shares of Neurogene OpCo Class A common stock were converted into 3,240,888 shares of the Company’s common stock, based on an exchange ratio of approximately 0.0756 (the “Exchange Ratio”).
- All of the then outstanding shares of Neurogene OpCo preferred stock were converted into 7,231,747 shares of the Company’s common stock, based on the Exchange Ratio. Refer to Note 12, *Preferred Stock*, for further detail on the conversion of Neurogene OpCo preferred stock.
- Each then outstanding Neurogene OpCo stock option was exchanged for an equivalent stock option of the Company, adjusted to reflect the Exchange Ratio as necessary.
- Each then outstanding Neurogene OpCo pre-funded warrant to purchase shares of Neurogene OpCo common stock was converted into a pre-funded warrant to purchase shares of the Company’s common stock, adjusted to reflect the Exchange Ratio as necessary. Refer to the discussion below for further detail on Neurogene OpCo pre-funded warrants.

Concurrently with the execution and delivery of the Merger Agreement, and in order to provide Neurogene OpCo with additional capital for its development programs, Neurogene OpCo entered into a subscription agreement (the “Subscription Agreement”) with certain investors. Pursuant to the terms of the Subscription Agreement, immediately prior to the Closing, Neurogene OpCo issued and sold to the investors: (i) 2,792,206 shares of Neurogene OpCo common stock and (ii) 1,811,739 pre-funded warrants, exercisable for 1,811,739 shares of Neurogene OpCo common stock, at a purchase price of approximately \$20.63 per share or \$20.63 per warrant, for an aggregate purchase price of approximately \$95.0 million (the “Pre-Closing Financing”).

On December 18, 2023, immediately prior to Closing, Neoleukin entered into a contingent value rights agreement (the “CVR Agreement”) with a rights agent and the lease representative, pursuant to which holders of Neoleukin common stock or pre-funded warrants prior to Closing received one non-transferable contingent value right (each, a “CVR”) for each outstanding share of Neoleukin common stock held by such stockholder or warrant holder immediately prior to Closing. Holders of options to purchase Neoleukin common stock outstanding immediately prior to the effective time of the reverse merger who elect to exercise those options following the reverse merger will also receive one CVR for each share of Neoleukin common stock issued upon exercise of such option, subject to certain conditions set forth in the CVR Agreement. Each CVR represents the contractual right to receive (i) certain net savings, if any, realized by the Company by June 30, 2029 in connection with Neoleukin’s legacy lease obligations (the “Lease CVR”), including those related to Neoleukin’s sublease, entered into in October 2023, (ii) 100% of net proceeds, if any, derived from any consideration paid as a result of the sale of Neoleukin’s pre-merger legacy assets pursuant to any agreements entered into before Closing, and 80% of net proceeds, if any, derived from any consideration paid as a result of the sale of Neoleukin’s pre-merger legacy assets pursuant to any agreements entered into within one year after Closing (the “Intellectual Property CVR”), and (iii) certain net proceeds, if any, derived from Neoleukin’s anticipated sales tax refund from Washington State relating to tax returns filed by Neoleukin prior to Closing (the “Sales Tax CVR”). As of December 31, 2023, the Company has recorded approximately \$0.3 million and \$1.0 million to lease contingent value rights liability, current and lease contingent value rights liability, non-current, respectively, related to amounts that are probable and estimable under the Lease CVR as of December 31, 2023. As no other amounts related to the CVR Agreement were probable as of December 31, 2023, no contingencies for the Intellectual Property CVR or Sales Tax CVR have been recorded. Refer to Note 10, *Commitments and Contingencies*, for further discussion on the CVR components.

Based on the following factors, the Company determined under the Accounting Standards Codification (“ASC”) 805, *Business Combinations*, that the merger should be accounted for as a reverse asset acquisition:

- Neurogene OpCo stockholders owned approximately 84% of the voting rights in the Company, and thus had sufficient voting rights to exert influence over the Company.
- Neurogene OpCo designated a majority of the Company’s board of directors and maintained a majority of the composition of management.
- At the time of Closing, Neoleukin did not meet the definition of a business but it did represent a group of assets.

For further discussion on the reverse merger and the related accounting, refer to Note 4, *Reverse Merger*.

## **2. Risks and Uncertainties**

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, successful development of technology, obtaining additional funding, protection of proprietary technology, compliance with government regulations, risks of failure of pre-clinical studies, clinical studies and clinical trials, the need to obtain marketing approval for its drug candidates and its consumer products, fluctuations in operating results, economic pressure impacting therapeutic pricing, dependence on key personnel, risks associated with changes in technologies, development by competitors of technological innovations and the ability to transition from pilot scale manufacturing to large scale production.

### ***Liquidity and Financial Condition***

Since its inception, the Company has funded its operations primarily with proceeds from the sales of equity securities and has incurred significant recurring losses, including net losses of \$36.3 million and \$55.2 million for the years ended December 31, 2023 and 2022, respectively. In addition, the Company used cash in operations of \$51.4 million and \$52.8 million for the years ended December 31, 2023 and 2022, respectively, and had an accumulated deficit of \$187.2 million as of December 31, 2023. Management expects to incur substantial and increasing losses in future periods as the Company advances its products through its clinical and regulatory process and will rely on outside capital to fund its operations for the foreseeable future. The Company has not generated

positive cash flows from operations, and there are no assurances that the Company will be successful in obtaining an adequate level of financing for the development and commercialization of its product candidates.

As of December 31, 2023, the Company had cash and cash equivalents of approximately \$148.2 million. On December 18, 2023, the Company closed on the merger with Neoleukin Therapeutics, Inc. (see Note 4, *Reverse Merger*) and a Pre-Closing Financing (see Note 1, *Organization and Description of Business*). The Company expects its available cash and cash equivalents on hand as of the issuance date of these financial statements will be sufficient to fund its obligations as they become due for at least one year beyond the issuance date of these financial statements. As a result of the reverse merger with Neoleukin Therapeutics, the Company assumed an ATM or “at-the-market” Equity Offering Sales Agreement (the “Sales Agreement”) with BofA Securities, Inc., as agent (“BofA”), pursuant to which the Company may offer and sell, from time to time through BofA, shares of the Company’s common stock, having an aggregate offering price of up to \$40.0 million. In March 2024, the Company terminated the ATM.

In the event the Company is unable to secure additional outside capital, management will be required to seek other alternatives which may include, among others, a delay or termination of clinical trials or the development of its product candidates, temporary or permanent curtailment of the Company’s operations, a sale of assets, or other alternatives with strategic or financial partners.

The accompanying financial statements do not include any adjustments that might result from the outcome of these uncertainties. Accordingly, the financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

### **3. Summary of Significant Accounting Policies**

#### ***Basis of Presentation***

The accompanying financial statements are presented in United States (“U.S.”) dollars and have been prepared in accordance with accounting principles generally accepted in the United States of America (“U.S. GAAP”).

#### ***Use of Estimates***

The preparation of the financial statements in accordance with US GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. In preparing these financial statements, management used significant estimates in the following areas, among others: recoverability of the Company’s net deferred tax assets and related valuation allowance, useful lives and recoverability of property and equipment, determining the Incremental Borrowing Rate (“IBR”) for calculating lease liabilities and related right-of-use (“ROU”) assets and finance lease assets, clinical trial accruals, accrual estimates for all CVRs, the value attributed to employee stock options and other stock-based awards and valuation of common stock. On an ongoing basis, the Company reviews its estimates to ensure that they appropriately reflect changes in the business or as new information becomes available. Actual results may differ from these estimates.

#### ***Segment Information***

Operating segments are defined as components of an entity for which separate financial information is available and that is regularly reviewed by the Chief Operating Decision Maker (“CODM”) in deciding how to allocate resources and in assessing performance. The Company’s CODM is its Chief Executive Officer. The Company operates as a single operating segment and has one reportable segment. The Company’s operations and its assets are held in the United States.

#### ***Cash and Cash Equivalents***

The Company considers all highly-liquid investments purchased with original maturities of 90 days or less at time of purchase to be cash equivalents. Cash and cash equivalents include cash held in banks and are stated at fair value.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash in the balance sheets that sum to the total of the same such amounts shown in the statements of cash flows (in thousands):

	December 31,	
	2023	2022
Cash and cash equivalents	\$ 148,210	\$ 82,021
Restricted cash	508	—
Total cash, cash equivalents and restricted cash	<u>\$ 148,718</u>	<u>\$ 82,021</u>

Cash equivalents consist of money market funds in which the carrying value equals the fair value (see Note 6, *Fair Value of Financial Instruments*). Restricted cash includes \$0.5 million in cash deposits the Company maintains with its bank as collateral for the irrevocable letters of credits related to its lease obligations.

#### ***Concentrations of Credit Risk***

Financial instruments that subject the Company to significant concentrations of credit risk consist primarily of cash and cash equivalents. The Company's cash and cash equivalent accounts, at times, may exceed federally insured limits. As of December 31, 2023, the Company had \$147.7 million in excess of the federally insured limits. The Company places its cash in financial institutions that management believes to be of high credit quality.

#### ***Investments***

Investment securities at December 31, 2023 consist of U.S. treasury notes. The Company classifies these securities as held-to-maturity. Held-to-maturity securities are those securities in which the Company has the ability and intent to hold the security until maturity. Held-to-maturity securities are recorded at amortized cost, adjusted for the amortization or accretion of premiums or discounts. Premiums and discounts are amortized or accreted over the life of the related held-to-maturity security as an adjustment to yield using the effective interest method. The Company reassesses the classification of held-to-maturity at each reporting period.

The allowance for credit losses on held-to-maturity securities is a contra-asset valuation account determined in accordance with ASC 326, which is deducted from the securities' amortized cost basis at the balance sheet date as a result of management's assessment of the net amount expected to be collected. Securities that are determined to be uncollectible are written off against the allowance. The Company did not recognize an allowance for credit losses as of December 31, 2023. Interest income is recognized when earned. Additional information regarding held-to-maturity investments is included in Note 5, *Investments*.

#### ***Fair Value of Financial Instruments***

Management believes that the carrying amounts of the Company's financial instruments, including cash, prepaid and other current assets, accounts payable and accrued expenses, approximate fair value due to the short-term nature of these instruments. Certain assets and liabilities are carried at fair value. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- **Level 1** – Unadjusted quoted prices in active markets that are accessible to the reporting entity at the measurement date for identical assets and liabilities.
- **Level 2** – Inputs other than quoted prices in active markets for identical assets and liabilities that are observable either directly or indirectly for substantially the full term of the asset or liability. Level 2 inputs include the following:
  - quoted prices for similar assets and liabilities in active markets.

- quoted prices for identical or similar assets or liabilities in markets that are not active.
- observable inputs other than quoted prices that are used in the valuation of the asset or liabilities (e.g., interest rate and yield curve quotes at commonly quoted intervals).
- inputs that are derived principally from or corroborated by observable market data by correlation or other means.
- **Level 3** – Unobservable inputs for the assets or liability (i.e., supported by little or no market activity). Level 3 inputs include management’s own assumptions about the assumptions that market participants would use in pricing the asset or liability (including assumptions about risk).

### ***Property and Equipment***

Property and equipment costs are stated at cost, net of accumulated depreciation and amortization. The cost of property and equipment costs are depreciated on the straight-line method over the following estimated useful lives:

Type	Estimated useful life
Lab equipment	5 years
Manufacturing equipment	10 years
Leasehold improvements	Lesser of the remaining economic life of the asset or the lease-term
Furniture and fixtures	5 years
Software	3 years

Leasehold improvements are amortized over the shorter of the estimated useful life of the assets or the remaining lease term. Major additions and betterments are capitalized; maintenance and repairs, which do not improve or extend the life of the respective assets, are charged to operating expenses as incurred. Depreciation has been calculated using the straight-line method over their estimated useful lives once the asset is placed in service. Costs of software obtained for internal use are capitalized in accordance with ASC 350 and are recognized on a straight-line basis over the useful life. Software costs that do not meet the capitalization criteria, including costs incurred in the maintenance and minor upgrade and enhancement of software without additional functionality, are expensed as incurred. No depreciation has been calculated on work in progress assets.

### ***Capitalized Software Development Costs***

Implementation costs incurred in a cloud computing arrangement that is a service contract are capitalized and recorded in prepaid expenses and other current assets and other non-current assets on the consolidated balance sheets. Capitalized implementation costs include external professional service costs related to technical development. Post-implementation training and maintenance costs are expensed as incurred. Capitalized eligible implementation costs related to cloud computing service contracts are included in prepaid expenses and other current assets and other non-current assets were \$0.3 million and \$0.4 million as of December 31, 2023, and 2022, respectively. Capitalized cloud computing implementation costs are amortized on a straight-line basis over the expected term of the arrangement. Expense recognized related to the implementation costs capitalized was \$0.2 million and \$0.2 million for the years ended December 31, 2023 and 2022, respectively. Cloud computing arrangements are tested for impairment whenever events or changes in circumstances occur that could impact the recoverability of these assets. No impairment of cloud computing arrangements occurred in 2023 or 2022.

### ***Impairment of Long-Lived Assets***

Management assesses the carrying value of property and equipment and software whenever events or changes in circumstances indicate that the carrying value may not be recoverable. If there is indication of impairment, management prepares an estimate of future undiscounted cash flows expected to result from the use of the asset and its eventual disposition. If these cash flows are less than the carrying amount of the asset, an impairment loss is

recognized to write down the asset to its estimated fair value. For the years ended December 31, 2023 and 2022, the Company did not recognize any impairments for its long-lived assets.

### **Leases**

Operating and finance leases are accounted for in accordance with ASU 2016-02, Leases as amended. They are presented in the Company's consolidated balance sheet as right-of-use assets from leases, current lease liabilities, and long-term lease liabilities. At the inception of a contractual arrangement, the Company determines whether the contract contains a lease by assessing whether there is an identified asset and whether the contract conveys the right to control the use of the identified asset in exchange for consideration over a period of time. If both criteria are met, the Company records the associated lease liability and corresponding ROU asset upon commencement of the lease using the implicit rate or a discount rate based on a credit-adjusted secured borrowing rate commensurate with the term of the lease. As the Company's leases do not provide an implicit rate, the Company utilizes the appropriate IBR, determined as the rate of interest that the Company would have to pay to borrow on a collateralized basis over a similar term and in a similar economic environment.

Both operating and finance lease assets represent a right to use an underlying asset for the lease term, and operating and finance lease liabilities represent an obligation to make lease payments arising from the lease. Lease liabilities with a term greater than one year and their corresponding ROU assets are recognized on the balance sheet at the commencement date of the lease based on the present value of lease payments over the expected lease term.

Certain of the Company's lease agreements at the adoption date contain renewal options. The Company does not recognize right-of-use assets or lease liabilities for renewal periods upon lease inception date unless it is determined that the Company is reasonably certain of renewing the lease at inception or when a triggering event occurs. The Company also made an accounting policy election to utilize the short-term lease exemption, whereby leases with a term of 12 months or less will not follow the recognition and measurement requirements of the standard.

The Company's leases do not contain any residual value guarantees. Since the Company elected to account for each lease component and its associated non-lease components as a single combined lease component, all contract consideration is allocated to the combined lease component. Some of the Company's lease agreements contain rent escalation clauses. For operating leases, the Company recognizes the minimum rental expense on a straight-line basis based on the fixed components of a lease arrangement. The Company will amortize this expense over the term of the lease beginning with the lease commencement date. Certain lease agreements contain variable payments, which are expensed as incurred and not included in the lease right-of-use assets and liabilities. These amounts include payments for maintenance and utilities. Variable lease components represent amounts that are not fixed in nature and are not tied to an index or rate and are recognized as incurred.

Additional information and disclosures are included in Note 10, *Commitments and Contingencies*.

### **Contingent Value Rights**

As discussed within the *Reverse Merger and Pre-Closing Financing* section in Note 1, *Organization and Description of Business*, the Company entered into a CVR Agreement on December 18, 2023 prior to Closing. Included in the CVR Agreement are three different types of CVRs: (i) the Lease CVR, (ii) the Intellectual Property CVR, and (iii) the Sales Tax CVR (together, the "CVRs"). The Company evaluated each of the CVRs to determine if they qualified as derivatives under ASC 815, *Derivatives and Hedging*, and concluded that since certain scope exceptions were met, the CVRs did not qualify as derivatives. Instead, the Company records a contingent consideration liability associated with the CVRs when payments are probable and estimable under ASC 450. In assessing whether payments are probable and estimable, the Company considers the existence of or ability to enter agreements with third parties or government agencies and timing of potential payments. As of December 31, 2023, the Company recorded \$0.3 million and \$1.0 million to lease contingent value rights liability, current and lease contingent value rights liability, non-current, respectively, related to amounts that are probable and estimable under the Lease CVR. As no other amounts related to the CVR Agreement were probable as of December 31, 2023, no contingencies for the Intellectual Property CVR or Sales Tax CVR have been recorded. Refer to Note 1,

*Organization and Description of Business*, and Note 10, *Commitments and Contingencies*, for further discussion on the CVR components.

#### ***Exit and Disposal Costs***

In connection with the reverse merger and through fiscal 2024, the Company has incurred and expects to incur costs to wind-down Neoleukin's Phase 1 NL-201 study. This study has ceased further development, and the Company has no plans to continue developing Neoleukin's *de novo* protein technology. As a result, the study's activities do not provide the Company any future economic benefit. In accordance with *ASC 420 Exit or Disposal Costs*, the Company accrued the remaining costs to be incurred in the contract of approximately \$0.9 million, which was included in the liabilities assumed in the reverse merger. As of December 31, 2023, no payments were made and the liability of \$0.8 million was classified as accrued expenses and other current liabilities in the consolidated balance sheet.

#### ***Research and Development***

Research and development costs are expensed as incurred. These costs include, but are not limited to, employee-related expenses, including salaries, benefits and travel of the Company's research and development personnel, facilities, supplies, rent, insurance, stock-based compensation, depreciation and external expenses incurred under agreements with contract research organizations and investigative sites that conduct preclinical studies and manufacture the drug product for the preclinical activities and other costs associated with preclinical activities.

Before a compound receives regulatory approval, the Company records upfront and milestone payments to third parties under licensing arrangements as expense provided that there is no alternative future use of the rights in other research and developments projects.

Non-refundable prepayments for research and development costs that are paid in advance of performance are capitalized as a prepaid expense and amortized over the service period as the services are provided. Costs for certain development activities, such as outside research programs funded by the Company, are recognized based on an evaluation of the progress to completion of specific tasks with respect to their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the financial statements as prepaid or accrued research and development expense as applicable. There can be judgment involved in measuring the research and development expenses to be recognized in a particular period. In some cases, expense is recorded using an underlying assumption of the progress to completion of specific activities. For example, costs may be recognized based on the passage of time for activities that span reporting periods. If the provision of services is not linear then this assumption could impact the amount of expense recognized. The level of judgment varies based on the nature of the services being performed and the underlying support obtained. For some activities, such as for certain clinical trials, expense is recorded based on information obtained from vendors as an intermediary to those performing the underlying services, such as contract research organizations. These estimates are inherently more judgmental since the quality and availability of the underlying data may vary. The Company does not need to make significant estimates where costs incurred are supported by invoices or reports of costs incurred are obtained from a vendor that is directly performing the underlying services, such as a consultant or contract manufacturing organization.

In-process research and development ("IPR&D") that is acquired through licensing arrangements and accounted for as asset acquisitions are expensed immediately and within research and development expenses if the IPR&D has no alternative future use.

#### ***General and Administrative***

General and administrative expenses consist primarily of personnel expenses, including salaries, benefits and stock-based compensation expense, for employees and consultants in executive, finance and accounting, legal, operations support, information technology and human resource functions. General and administrative expenses also include corporate facility costs not otherwise included in research and development expense, including rent, utilities,

depreciation and maintenance, as well as legal fees related to intellectual property and corporate matters and fees for accounting and consulting services.

### ***Stock-Based Compensation***

The Company accounts for stock-based compensation awards in accordance with ASC Topic 718, Compensation – Stock Compensation (“ASC 718”). ASC 718 requires all stock-based payments, including grants of stock options and restricted stock, to be recognized in the consolidated statements of operations based on their fair values. All of the stock-based awards are subject only to service-based vesting conditions. Management estimates the fair value of the stock option awards using the Black-Scholes option pricing model, which requires the input of assumptions, including (a) the fair value of the Company’s common stock, (b) the expected stock price volatility, (c) the calculation of expected term of the award, (d) the risk-free interest rate and (e) expected dividends. Management estimates the fair value of the restricted stock awards using the fair value of the Company’s common stock. Forfeitures are recognized as they are incurred.

Prior to the reverse merger, the Company periodically estimated the fair value of the Company’s common stock considering, among other things, valuations of its common stock prepared by management with the assistance of a third party valuation firm in accordance with the guidance provided by the American Institute of Certified Public Accountants 2013 Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. Following the reverse merger, the fair value of the Company’s common stock is based on the closing stock price on the date of grant as reported on the Nasdaq Global Market. The expected life of the stock options in years is estimated using the “simplified method,” as prescribed in SEC’s Staff Accounting Bulletin (SAB) No. 107, as the Company has no historical information from which to develop reasonable expectations about future exercise patterns and post-vesting employment termination behavior for its stock option grants. The simplified method is the midpoint between the vesting period and the contractual term of the option. For stock price volatility, the Company uses comparable public companies as a basis for its expected volatility to calculate the fair value of option grants. The risk-free rate is based on the U.S. Treasury yield curve commensurate with the expected life of the option. The expected dividend yield is zero as the Company has no history of paying dividends and no plans to do so in the near term.

The Company classified stock-based compensation expense in its consolidated statement of operations in the same manner of the award recipient’s payroll costs.

### ***Income Taxes***

The Company accounts for income taxes in accordance with ASC 740, *Income Taxes*, which prescribes the use of the liability method whereby deferred tax assets and liabilities are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is provided to reduce the net deferred tax assets to a level which, more likely than not, will be realized.

The Company assesses its income tax positions and records tax benefits based upon management’s evaluation of the facts, circumstances, and information available at the reporting date. For those tax positions where it is more likely than not that a tax benefit will be sustained, the Company records the amount of tax benefit with a greater than 50 percent likelihood of being realized upon ultimate settlement with a taxing authority having full knowledge of all relevant information. For those income tax positions for which it is not more likely than not that a tax benefit will be sustained, no tax benefit is recognized in the financial statements. Potential interest and penalties associated with such uncertain tax positions is recorded as a component of income tax expense.

### ***Net Income (Loss) Per Share***

For the prior year and pre-merger periods in which the Company had multiple classes of stock participating in earnings, the Company uses the two-class method in calculating earnings per share. Basic earnings per share of Class A and Class B common stock is computed by dividing net income attributable to Neurogene OpCo by the weighted-average number of shares of Class A and Class B common stock outstanding during the period.



For the post-merger period, the only period in which the Company had income, diluted earnings per share of the single class of common stock is computed by dividing net income attributable to the Company, adjusted for the assumed exchange of all potentially dilutive instruments for common stock, by the weighted-average number of shares of common stock outstanding, adjusted to give effect to potentially dilutive elements.

As of December 18, 2023, the Company consummated the reverse merger, leaving only one class of common stock remaining, and as such, the two-class method is not presented for the post-merger period from December 18, 2023 to December 31, 2023. The single class of common stock remaining post-merger is referred to throughout as “common stock.”

The following table sets forth the computation of basic and diluted net income (loss) per share (in thousands, except share and per share amounts):

	Year Ended December 31, 2023			Year Ended December 31, 2022	
	Pre-Merger <sup>(1)</sup>		Post-Merger <sup>(2)</sup>	Pre-Merger <sup>(1)</sup>	
	Class A	Class B	Common stock	Class A	Class B
Net income (loss) per share, basic:					
Numerator:					
Net income (loss)	\$ (49,971)	\$ —	\$ 13,654	\$ (55,189)	\$ —
Denominator:					
Weighted-average shares outstanding used in computing net income (loss) per share, basic	426,097	—	491,867	394,533	—
Net income (loss) per share, basic	<u>\$ (117.28)</u>	<u>\$ —</u>	<u>\$ 27.76</u>	<u>\$ (139.88)</u>	<u>\$ —</u>
Net income (loss) per share, diluted:					
Numerator:					
Net income (loss)	\$ (49,971)	\$ —	\$ 13,654	\$ (55,189)	\$ —
Denominator:					
Weighted-average shares outstanding used in computing net income (loss) per share, basic	426,097	—	491,867	394,533	—
Weighted-average effect of dilutive securities:					
Warrants to purchase common stock	—	—	4,063,361	—	—
Options to purchase common stock	—	—	101,719	—	—
Weighted-average shares outstanding used in computing net income (loss) per share, diluted	426,097	—	4,656,947	394,533	—
Net income (loss) per share, diluted	<u>\$ (117.28)</u>	<u>\$ —</u>	<u>\$ 2.93</u>	<u>\$ (139.88)</u>	<u>\$ —</u>

<sup>(1)</sup> Represents the period starting January 1, 2023 through Closing for the year ended December 31, 2023 and for the year ended December 31, 2022.

<sup>(2)</sup> Represents the period subsequent to Closing (December 18, 2023) through December 31, 2023.

Potentially dilutive securities that were not included in the diluted per share calculations because they would be antidilutive were as follows:

	Year Ended December 31, 2023			Year Ended December 31, 2022	
	Pre-Merger <sup>(1)</sup>		Post-Merger <sup>(2)</sup>	Pre-Merger <sup>(1)</sup>	
	Class A	Class B	Common stock	Class A	Class B
Options to purchase common stock	614,729	—	541,180	468,632	—
Restricted stock awards	—	—	—	6,691	—
Series A-1 convertible preferred stock <sup>(3)</sup>	—	—	—	18,604,653	—
Series A-2 convertible preferred stock <sup>(3)</sup>	—	—	—	13,291,208	—
Series B convertible preferred stock <sup>(3)</sup>	—	—	—	74,405,719	—
Total awards excluded	614,729	—	541,180	106,776,903	—

<sup>(3)</sup> The convertible preferred stock does not reflect the application of the 0.0756 Exchange Ratio.

#### **Recently Issued Accounting Standards**

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies that the Company adopts as of the specified effective date. Unless otherwise discussed below, the Company does not believe that the adoption of recently issued standards have or may have a material impact on our financial statements or disclosures.

#### **Recently Issued Accounting Pronouncements Not Yet Adopted**

In November 2023, the FASB issued ASU 2023-07, Segment Reporting (Topic 280). The amendments in this update expand segment disclosure requirements, including new segment disclosure requirements for entities with a single reportable segment among other disclosure requirements. This update is effective for the Company in the consolidated financial statements for the year ending December 31, 2024, and interim periods beginning after January 1, 2025. The Company is analyzing the impact of this standard on its disclosures in the consolidated financial statements.

In December 2023, the FASB issued ASU 2023-09, Improvements to Income Tax Disclosures which requires consistent categories and greater disaggregation of information in the rate reconciliation and income taxes paid disaggregated by jurisdiction. This standard will be effective for annual periods beginning after December 15, 2024. The Company is currently evaluating the impact of this standard on the Company's consolidated financial statements and related disclosures but does not expect the adoption of ASU 2020-09 to be material.

#### **Recently Adopted Accounting Pronouncements**

The Company adopted ASU 2016-13, *Financial Instruments - Credit Losses*, as amended, on January 1, 2023. This ASU sets forth a current expected credit loss model which requires the Company to measure all expected credit losses for financial instruments held at the reporting date based on historical experience, current conditions, and reasonable supportable forecasts. This replaces the existing incurred loss model and is applicable to the measurement of credit losses on financial assets measured at amortized cost and applies to some off-balance sheet credit exposures. The impact of ASU 2016-13 on the Company's consolidated financial statements was not material.

#### **4. Reverse Merger**

As discussed within the *Reverse Merger and Pre-Closing Financing* section in Note 1, *Organization and Description of Business*, on December 18, 2023 the reverse merger between Neoleukin, Merger Sub, and Neurogene OpCo was consummated. Pursuant to the Merger Agreement, upon Closing, Merger Sub merged with and into Neurogene OpCo, with Neurogene OpCo surviving as a wholly owned subsidiary and surviving corporation of the

merger. In connection with the completion of the reverse merger, the Company changed its name from “Neoleukin Therapeutics, Inc.” to “Neurogene Inc.,” and the business conducted by the Company became primarily the business conducted by Neurogene OpCo. The reverse merger was contemplated and consummated, along with the Pre-Closing Financing, to generate capital resources to support the advancement of the Company’s pipeline and future operations.

As further discussed in Note 1, *Organization and Description of Business*, the reverse merger was accounted for as a reverse asset acquisition pursuant to ASC 805, *Business Combinations* as Neoleukin did not meet the definition of a business under ASC 805 but it did represent a group of assets. Therefore, the transaction was treated as the equivalent of Neurogene issuing stock to acquire the net assets of Neoleukin. In accordance with the relative fair value allocation guidance in ASC 805, to account for the reverse merger, the Company allocated the difference between the total consideration transferred and the fair value of the net assets acquired to Neoleukin’s non-monetary assets on a pro rata basis. As the fair value of Neoleukin’s net assets acquired at Closing exceeded the total consideration transferred after the fair value allocation, (i) \$1.3 million of the difference was recorded as a contingent consideration liability on the Company’s consolidated balance sheet for payments that are probable and estimable under the CVR Agreement and (ii) the remaining \$16.4 million was recognized as a bargain purchase gain in the Company’s consolidated statement of operations.

The following table summarizes the final purchase price paid in the reverse merger along with the fair value allocation (in thousands, except share and per share amounts):

<b>Calculation of total purchase price:</b>	
Number of common shares and pre-funded warrants of the combined company owned by Neoleukin stockholders <sup>(1)</sup>	2,777,017
Multiplied by the fair value per share of Neoleukin common stock <sup>(2)</sup>	\$ 13.76
Fair value of Neoleukin common stock issued <sup>(2)</sup>	\$ 38,212
Transaction costs <sup>(6)</sup>	4,140
Contingent consideration liability <sup>(3)</sup>	1,287
<b>Total purchase price</b>	<b>\$ 43,639</b>
<b>Fair value of Neoleukin net assets acquired at Closing and fair value allocation:</b>	
Monetary assets acquired <sup>(4)</sup>	\$ 76,906
Liabilities assumed <sup>(5)</sup>	(21,052)
Fair value of net assets acquired	\$ 55,854
Less: total consideration transferred	(38,212)
Excess of fair value of monetary assets acquired over total consideration transferred	\$ 17,642
Fair value allocated to contingent consideration liability <sup>(3)</sup>	1,287
Fair value allocated to bargain purchase gain	16,355
<b>Total fair value allocated</b>	<b>\$ 17,642</b>
<b>Reconciliation of allocation to purchase price:</b>	
Fair value of monetary assets acquired <sup>(4)</sup>	\$ 76,906
Transaction costs <sup>(6)</sup>	4,140
Neoleukin liabilities assumed <sup>(5)</sup>	(21,052)
Fair value allocated to bargain purchase gain	(16,355)
<b>Total purchase price</b>	<b>\$ 43,639</b>

(1) The number of shares is based on a total 2,351,030 shares of Neoleukin common stock and 425,987 pre-funded warrants outstanding as of December 18, 2023.

- (2) The fair value of Neoleukin common stock (also referred to in discussions herein as “total consideration transferred”) was determined using the closing price of Neoleukin’s common stock on December 15, 2023, after giving effect to the Reverse Stock Split. As discussed in Note 3, *Summary of Significant Accounting Policies*, Neoleukin’s stock price is measured using Level 1 fair value measurements.
- (3) Refer to Note 10, *Commitments and Contingencies*, for further discussion on the contingent consideration liability.
- (4) In accordance with the guidance in ASC 805, *Business Combinations*, the fair value of the net assets acquired was allocated to any non-monetary assets on a pro rata basis. As such, the assets acquired only include cash, cash equivalents and restricted cash of \$22.2 million, short-term investments of \$53.9 million, and other immaterial monetary assets of \$0.8 million.
- (5) Included in Neoleukin liabilities assumed is (i) \$10.9 million in lease liabilities, (ii) \$3.8 million in accrued payroll and \$6.3 million in other accrued costs and accounts payable. For the lease liabilities, the Company utilized judgment in estimating the IBR used in determining the fair value of the leases upon Closing.
- (6) As indicated in ASC 805 regarding asset purchases, the accounting acquirer’s transaction costs incurred directly related to the asset purchase should be included in the consideration to acquire the assets. These costs were initially recorded as deferred financing costs and then reclassified to offset to equity upon Closing.

## 5. Investments

The following table summarizes the Company’s investment securities as of December 31, 2023 (in thousands):

	December 31, 2023			
	Amortized cost, as adjusted	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value
<b>Short-term investments:</b>				
U.S. treasury notes	\$ 48,947	\$ 16	\$ —	\$ 48,963

	December 31, 2022			
	Amortized cost, as adjusted	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value
<b>Short-term investments:</b>				
U.S. treasury notes	\$ —	\$ —	\$ —	\$ —

All of the Company’s investments mature within the next 12 months.

## 6. Fair Value of Financial Instruments

As of December 31, 2023 and December 31, 2022, financial assets measured at fair value on a recurring basis are categorized in the table below based upon the lowest level of significant input to the valuations (in thousands):

	December 31, 2023		
	Level 1	Level 2	Level 3
<b>Assets:</b>			
Money market funds	\$ 144,358	\$ —	\$ —
U.S. treasury notes	48,947	—	—
<b>Total</b>	<b>\$ 193,305</b>	<b>\$ —</b>	<b>\$ —</b>

	December 31, 2022		
	Level 1	Level 2	Level 3
<b>Assets:</b>			
Money market funds	\$ 78,749	\$ —	\$ —

Money market funds are cash equivalents and are included in cash and cash equivalents in the consolidated balance sheet as of December 31, 2023 and 2022.

## 7. Prepaid expenses and other current assets

Prepaid expenses and other assets consist of the following (in thousands):

	December 31,	
	2023	2022
Refunds and other receivables	\$ 600	\$ 990
Prepaid expenses	1,496	1,078
Other current assets	1,095	630
Total prepaid and other current assets	<u>\$ 3,191</u>	<u>\$ 2,698</u>

## 8. Property and Equipment, Net

Property and equipment consist of the following (in thousands):

	December 31,	
	2023	2022
Lab equipment	\$ 3,144	\$ 3,088
Manufacturing equipment	6,142	5,955
Leasehold improvements	15,376	15,298
Office equipment	19	—
Software	268	289
Construction in progress	234	252
Total property and equipment, cost	<u>25,183</u>	<u>24,882</u>
Less accumulated depreciation and amortization	(8,009)	(4,767)
Property and equipment, net	<u>\$ 17,174</u>	<u>\$ 20,115</u>

Depreciation and amortization expense for the years ended December 31, 2023 and December 31, 2022 was approximately \$3.3 million and \$3.2 million, respectively.

## 9. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consist of the following (in thousands):

	December 31,	
	2023	2022
Compensation and related benefits	\$ 3,496	\$ 3,357
Research and development	4,895	1,800
Accrued severance, bonus, and retention <sup>(1)</sup>	2,476	—
Accrued offering costs in connection with pre-closing financing	3,334	—
Accrued transaction costs related to reverse merger	2,557	—
Other	737	167
Total accrued expenses and other current liabilities	<u>\$ 17,495</u>	<u>\$ 5,324</u>

(1) Includes accrued severance, bonus, and retention payments for current and former Neoleukin employees. Refer to Note 10, Commitments and Contingencies, for further detail.

## 10. Commitments and Contingencies

### *Finance Leases*

During the years ended December 31, 2023 and 2022, the Company leased information technology (“IT”) equipment and copiers for periods ranging from 36 months to 63 months. The IT equipment were classified as finance leases due to the existence of bargain purchase options in the lease agreements, and the copiers were classified as finance leases as the lease periods represented a major portion of the economic life of those assets. In connections with these leases, the Company recorded finance lease right-of-use assets and finance lease liabilities of approximately \$0.1 million.

### *Operating Leases*

#### *New York Headquarters Lease*

In September 2019, the Company commenced a sub-lease of approximately 6,000 square feet of office space for the corporate headquarters in New York, New York with a term expiring in June 2023. In connection with the lease, the Company established an irrevocable letter of credit for approximately \$0.4 million. Monthly lease payments were approximately \$0.04 million.

In July 2021, the sublessor was released from the original lease by the landlord, and the Company attorned to the landlord the executory terms and provisions of the sub-lease. In February 2022, the Company entered into an extension of the New York office lease (retroactive to December 2021) through June 2026, with new monthly lease payments ranging from approximately \$0.03 million to \$0.04 million. The Company accounted for the amendment as a contract modification, and accordingly, recorded an additional operating right-of-use asset of approximately \$1.0 million and an additional operating lease liability of approximately \$1.0 million.

#### *Houston Lease*

In August 2019, the Company entered into an agreement to lease approximately 26,905 square feet in Houston, Texas to build a manufacturing facility and office with a term expiring in August 2029. The Company has the option to renew the lease term for two additional five-year terms. The renewal periods were not included in the lease term for purposes of determining the lease liability or right-of-use asset. Monthly rent payments were approximately \$0.03 million. In connection with the lease, the Company paid a security deposit of approximately \$0.04 million and prepaid rent of approximately \$0.04 million.

In September 2020, the Company amended the lease agreement to further increase the rentable space to 42,342 square feet. The commencement date of the expansion space lease was January 1, 2021 and the monthly rent payments increased to a range of approximately \$0.05 million to \$0.06 million.

#### *Blaine Lease in Seattle*

As a result of the reverse merger, the Company assumed an operating lease for approximately 33,300 square feet of office space in Seattle, Washington for offices, a laboratory for research and development, and related uses. The lease expires on February 1, 2029, with the option to extend the lease for two five-year terms. The renewal periods were not included in the lease term for purposes of determining the lease liability or right-of-use asset.

#### *Eastlake Lease in Seattle*

As a result of the reverse merger, the Company assumed an operating lease for approximately 6,272 square feet of office space in Seattle, Washington, for additional office and laboratory space for research and development and related uses (the “Eastlake Lease”). The Company also assumed the existing agreement to sublease the Eastlake Lease to an unrelated third party (“Eastlake Sublease”). Pursuant to the terms of the EastLake Sublease, Neurogene is entitled to receive approximately \$1.6 million in lease payments. The term of the sublease is through September 30, 2026.

Supplemental lease expense related to leases for the years ended December 31, 2023 and 2022 was as follows (in thousands):

	Years Ended December 31,	
	2023	2022
Operating lease cost	\$ 1,037	\$ 1,037
Finance lease cost		
Amortization of finance leases	35	4
Interest on finance lease liabilities	12	2
Variable lease cost	178	196
Short-term lease cost	80	74
Total lease cost	<u>\$ 1,342</u>	<u>\$ 1,313</u>

The calculation of the present value of the lease payments for operating leases did not include any options to extend the leases as the Company is not reasonably certain to exercise such options.

The following table summarizes the maturity of the Company's operating and finance lease liabilities on an undiscounted cash flow basis and a reconciliation to the operating and finance lease liabilities recognized on the Company's consolidated balance sheet as of December 31, 2023 (in thousands):

<b>Maturity of operating lease liabilities</b>		
2024	\$	3,862
2025		3,964
2026		3,672
2027		3,235
2028		3,294
2029		616
Total lease payments	\$	18,643
Less: interest		(3,782)
Total operating lease liabilities	<u>\$</u>	<u>14,861</u>

<b>Maturity of finance lease liabilities</b>		
2024	\$	51
2025		50
2026		15
2027		6
2028		1
Total lease payments	\$	123
Less: interest		(16)
Total finance lease liabilities	<u>\$</u>	<u>107</u>

Supplemental balance sheet information related to leases as of December 31, 2023 and 2022 was as follows (in thousands):

Leases	December 31,	
	2023	2022
Operating right-of-use assets	\$ 3,681	\$ 4,344
Operating current lease liabilities	2,559	678
Operating noncurrent lease liabilities	12,302	3,921
Total operating lease liabilities	\$ 14,861	\$ 4,599
Finance right-of-use assets	\$ 98	\$ 87
Finance current lease liabilities	42	24
Finance noncurrent lease liabilities	65	66
Total finance lease liabilities	\$ 107	\$ 90
<b>Other information</b>	<b>December 31,</b>	
	<b>2023</b>	<b>2022</b>
Cash paid for amounts included in measurement of operating lease liabilities (in thousands)	\$ 1,051	\$ 1,039
Cash paid for amounts included in measurement of finance lease liabilities (in thousands)	\$ 42	\$ 3
Weighted-average remaining lease term - operating leases (in years)	4.88	5.72
Weighted-average remaining lease term - finance lease (in years)	2.53	3.47
Weighted-average discount rate - operating leases	9.72 %	8.86 %
Weighted-average discount rate - finance lease	11.49 %	11.45 %

#### **Lease CVR**

In accordance with the terms of the Lease CVR within the CVR Agreement, the Company accrued for approximately \$1.3 million as a contingent consideration liability on its consolidated balance sheet. As of the Closing, the expected range for the Lease CVR was approximately \$1.3 million to \$19.5 million based on the potential rights to CVR holders as described in the terms of the CVR Agreement.

As of December 31, 2023, \$1.3 million was recorded on the Company's consolidated balance sheet as a lease contingent value rights liability consisting of lease commitments that were probable and estimable as the Closing. The commitments relate to Neoleukin's sublease agreement, effective October 31, 2023, for one of its properties with an unrelated third party for the remainder of the lease term. All other payments under the CVR Agreement were not considered probable and estimable as of December 31, 2023 and therefore no additional contingent consideration liability has been recorded.

The Company will evaluate the probable and estimable range of outcomes under the CVR Agreement at each reporting period until the end of the CVR term and adjust the amounts accrued for as necessary.



The following table summarizes the maturity of the Company's lease CVR as of December 31, 2023 (in thousands):

<b>Maturity of Lease CVR</b>		
2024	\$	281
2025		598
2026		408
Total lease CVR payments	\$	<u>1,287</u>

#### ***Intellectual Property CVR***

Prior to the Closing, Neoleukin entered into a licensing agreement on December 13, 2023 with Accipiter Biosciences ("Accipiter") to develop and commercialize Neoleukin's *de novo* protein technology (the "Accipiter Licensing Agreement"). As discussed and defined within the *Reverse Merger and Pre-Closing Financing* section of Note 1, *Organization and Description of Business*, the terms of the CVR Agreement include that CVR holders are eligible to receive the Intellectual Property CVR. In addition, as discussed in Note 3, *Summary of Significant Accounting Policies*, contingent consideration liabilities related to the CVR Agreement will only be recorded if they are probable and estimable as of the balance sheet date.

Under the Accipiter Licensing Agreement, Accipiter will make a non-refundable up-front payment of approximately \$0.2 million upon the earlier of six months after the effective date or within 30 days after Accipiter closes on a seed financing of at least \$5.0 million. If Accipiter does not make the upfront payment within six months after the effective date, this agreement will terminate and neither party shall have any obligation to the other party. In addition, the Accipiter Licensing Agreement contains development, regulatory and commercialization milestones totaling up to approximately \$13.4 million, as well as royalty payments.

Since the upfront payment, milestones and royalties were not considered probable as of December 31, 2023, the Company did not record a contingent consideration liability related to the Intellectual Property CVR.

#### ***Employment Agreements***

Neoleukin entered into employment agreements with key personnel providing for severance payments, benefits, and other employee related costs in certain circumstances, as defined in the respective employment agreements. As of December 31, 2023, \$2.5 million of accrued severance, bonus, and retention costs were recorded to accrued expenses and other current liabilities on the Company's consolidated balance sheet as noted in Note 9, *Accrued Expenses and Other Current Liabilities*.

#### ***Other Research and Development Arrangements***

The Company enters into agreements with contract research organizations ("CROs") to assist in the performance of research and development activities. Expenditures to CROs will represent a significant cost in clinical development for the Company.

#### ***Litigation and Legal Proceedings***

The Company is subject to litigation and other claims that arise in the ordinary course of business. While the ultimate result of outstanding legal matters cannot presently be determined, the Company does not expect that the ultimate disposition will have a material effect on its results of financial condition, results of operations or cash flows. However, legal matters are inherently unpredictable and subject to significant uncertainties, some of which are beyond the Company's control. As such, there can be no assurance that the final outcome of any particular legal matter will not have a material adverse effect on the Company's financial condition, results of operations or cash flows.

## 11. License Agreements

### *License Agreement with The University of North Carolina*

In May 2019, Neurogene entered into an Exclusive License Agreement with the University of North Carolina at Chapel Hill (“UNC”) to obtain an exclusive, worldwide, royalty bearing license, with the right to grant sublicenses under certain patents to make, use, or sell products covered by such patents for prevention or treatment of disease or medical or genetic conditions, including CLN5 Batten disease or other diseases from dysfunction of the CLN5 gene. The Company is obligated to pay UNC up to \$1.7 million in sales-related milestones for licensed products based on annual sales of the licensed product in excess of defined thresholds and low single-digit percentage royalties on net sales of licensed product for as long as there is a valid patent claim under the patent rights. Neurogene is also required to reimburse any patent expenses, as well as pay a nonrefundable annual maintenance fee which, when royalties become due and payable, will be creditable against such royalties. The annual license fee was \$4,000 for each of the years ended December 31, 2023 and 2022.

### *License Agreement with The University of Edinburgh*

In January 2020, Neurogene entered into an Option Agreement (the “Edinburgh Option Agreement”) with the University Court of the University of Edinburgh (“University of Edinburgh”) for an option to license certain patents covering the EXACT technology (the “Licensed Technology”). To secure the option, Neurogene was solely required to pay the costs associated with the filing, preparing, prosecution and maintenance of the patents covering the Licensed Technology during the option period. No other payments were payable under the Edinburgh Option Agreement. Neurogene subsequently exercised the option under the Edinburgh Option Agreement and then entered into the Master Collaboration Agreement (“MCA”) discussed below, and which superseded the Edinburgh Option Agreement.

In December 2020, University of Edinburgh and Neurogene entered into the MCA. Under the MCA, Neurogene and University of Edinburgh agreed to collaborate on certain research and development projects (“Projects”) and Neurogene agreed to provide funding for such Projects for a 40-month initial term, which term may be extended by mutual agreement. In exchange for such funding, University of Edinburgh granted Neurogene the option to exclusively license any intellectual property arising from such Projects. If Neurogene exercises an exclusive option for a particular Project, Neurogene will enter into a separate exclusive license agreement on its own terms with University of Edinburgh. Under the MCA, Neurogene is obligated to pay semi-annual installment payments relating to funding of costs for personnel and lab consumables for the 40-month period. Either party may terminate the MCA for convenience upon 90 days’ notice. If Neurogene terminates the MCA, it would be responsible for all non-cancellable costs and commitments related to any particular Project and any and all funding costs for any person working on such Project. The expense recorded for the years ended December 31, 2023 and 2022 was \$1.6 million and \$1.1 million, respectively.

In March 2022, Neurogene exercised its option through the collaboration under the MCA, and entered into a License Agreement (the “March 2022 Edinburgh License Agreement”) with University of Edinburgh, pursuant to which Neurogene licensed certain patents and know-how related to the EXACT technology and optimized MECP2 cassettes on an exclusive basis. Under the March 2022 Edinburgh License Agreement, Neurogene obtained an exclusive, worldwide license to the licensed patents to develop, manufacture, supply, sell, and commercialize any products that utilize the licensed patents (the “Licensed Products”) in exchange for low single-digit percentage royalties on future commercial net sales of the Licensed Products. Royalties are payable on a Licensed Product-by-Licensed Product and country-by-country basis until the latter of the expiration of the last licensed patent covering such Licensed Product in the country where the Licensed Product is sold, or, if no licensed patent exists or has expired in such country, then ten years from first commercial sale of such Licensed Product in such country. In connection with the license, Neurogene is also obligated to pay the University of Edinburgh up to \$5.25 million in regulatory-related milestones and up to \$25 million in sales-related milestones based on annual net sales of Licensed Products in excess of defined thresholds. During the year ended December 31, 2023, the Company expensed \$0.3 million for a milestone related to the first patient dosing in the Phase 1/2 Rett study.

In November 2023, Neurogene and University of Edinburgh amended the MCA. Under the amended MCA, Neurogene and University of Edinburgh agreed to continue collaborating on certain Projects and Neurogene agreed to provide funding for such Projects through December 2026 or an additional 33 months. Neurogene is obligated to pay semi-annual installment payments relating to funding of costs for personnel and lab consumables for the entire period.

***License Agreement with the University of North Carolina and University of Pennsylvania***

In July 2020, the Company entered into an exclusive license agreement with the University of North Carolina and University of Pennsylvania, or the Universities, to further develop and commercialize the licensed technology for the Optimized GALC Genes and Expression Cassettes. The Company also has the right to sublicense the technology. The Company made an upfront payment to the Universities of \$0.5 million that was immediately expensed within research and development expenses as the license has no alternative future use. On an on-going basis, the Company is obligated to pay for future patent costs incurred, and such costs were immaterial for the years ended December 31, 2023 and December 31, 2022. During the year ended December 31, 2022, the Company paid the University of North Carolina a \$0.5 million milestone payment after receipt of the Rare Pediatric Disease Designation of NGN-201 from the FDA for Krabbe disease. An annual license maintenance fee is also payable commencing on the first anniversary of the effective date. The amount of the license fee was approximately \$0 and \$0.02 million for the years ended December 31, 2023 and 2022, respectively. In October 2023, the Company terminated the license agreement.

***License Agreement with Virovek***

In September 2020, Neurogene entered into a Non-Exclusive License Agreement with Virovek, Inc., pursuant to which Neurogene has a license to use certain patents and know-how on a non-exclusive basis related to Neurogene's baculovirus ("baculo") process in exchange for low single-digit percentage royalties on future commercial net sales of each product using the baculo process, development milestone payments of up to \$0.2 million in the aggregate, and a nonrefundable annual license fee. During the year ended December 31, 2023, the Company recorded a milestone expense of \$0.1 million for first filing of the IND filed in connection with the Company's Rett syndrome program. The license fee expense for each of the years ended December 31, 2023 and 2022 was \$0.05 million.

***License Agreement with Sigma-Aldrich Co***

In January 2023, Neurogene entered into a Non-Exclusive License Agreement with Sigma-Aldrich Co. LLC, pursuant to which Neurogene has a license to certain patents and know-how on a non-exclusive basis related to certain cell lines used in Neurogene's baculo process in exchange for a small annual fee on a product-by-product basis, payable once the first product candidate enters the clinic. In addition, on a product-by-product basis, Neurogene is obligated to pay up to \$2.5 million in the aggregate for development-related milestones. During the year end ended December 31, 2023, the Company recorded the expense for the initial annual license fee of approximately \$0.06 million.

No expenses were recorded related to other in-process license agreements during the years ended December 31, 2023 and 2022, respectively. None will be due under these agreements unless and until certain development milestones are reached.

**12. Preferred Stock**

***Preferred Stock***

The Company is authorized to issue 50,000,000 shares of preferred stock with a par value of \$0.000001 per share as of December 31, 2023. No shares are outstanding as of December 31, 2023.

### ***Convertible Preferred Stock***

On March 2, 2022, the Company entered into the Series B preferred stock purchase agreement, which provided for the purchase of 27,274,586 shares of Series B preferred stock, at \$2.44 per share for net proceeds of approximately \$66.5 million, of which the Company received such funds from March 2022 through June 2022.

On December 18, 2023, Neurogene OpCo completed the reverse merger with Neoleukin. Neurogene OpCo's Series A-1, Series A-2 and Series B preferred stock (collectively, "convertible preferred stock") was subject to certain pre-existing anti-dilution provisions that were triggered upon the closing of the Pre-Closing Financing. The private placement in Neurogene OpCo closed immediately before the Closing. As a result of the pre-existing anti-dilution feature, Neurogene OpCo preferred shareholders received an additional 13,505,271 shares of preferred stock. At the effective time of the reverse merger, each issued and outstanding share of Neurogene OpCo convertible preferred stock converted automatically into 0.0756 shares of the Company's common stock or pre-funded warrants, at the option of the preferred shareholder. At the Closing, the Company issued an aggregate of 7,231,747 shares of its common stock and 1,825,635 pre-funded warrants to Neurogene OpCo preferred shareholders.

Prior to the effective time of the reverse merger, the convertible preferred stock had the followings rights and privileges:

#### ***Dividends***

The holders of convertible preferred stock were entitled to receive non-cumulative dividends that would accrue at the rate of \$0.18 per share per year, payable only when and if declared by the Board of Directors. The Company would not declare, pay or set aside any dividends on shares of any class of common stock (as defined below) unless the holders of the convertible preferred stock would first receive dividends on each outstanding share of preferred stock in the amount of the accrued dividends unpaid as of such date. No dividends were issued for the convertible preferred stock from inception through the Closing.

#### ***Liquidation***

In the event of any liquidation, dissolution, or winding-up of the Company, which would include the sale of the Company, the convertible preferred stock was senior to common stock. The preferred shareholders would be entitled to preferential payment in the amount per share equal to the greater of (i) the original issue price and accrued dividends declared and unpaid or (ii) the amount that would be due had all convertible preferred stock been converted to common stock immediately prior to a deemed liquidation event. Upon payment of the preferred liquidation preference payments, the holders of Series A-1 and common stock participate on a pro-rata basis until the A-1 stockholders have received a liquidation preference amount of \$5.38 per share of Series A-1. Any remaining distribution thereafter would be distributed to holders of common stock.

#### ***Voting***

The preferred stockholders were entitled to the number of votes equal to the number of Class A common stock into which the shares of convertible preferred stock Series A and B held by each holder are then convertible.

#### ***Conversion***

The preferred stockholders had the option to convert each share of convertible preferred stock into Class A or Class B common stock, as applicable, at any time, and without additional payment. The number of Class A or Class B common stock into which the convertible preferred stock converts was equal to the original issuance price (defined as \$2.15 per share for the Series A and \$2.44 per share for the Series B) divided by the conversion price. The conversion price would initially be \$2.15 per share for the Series A and \$2.44 per share for the Series B and could be adjusted for certain dilutive events such as a down-round provision, stock splits and combinations, certain dividends and distributions or any merger or reorganization. Conversion to Class A common stock would have been mandatory upon the closing of an initial public offering resulting in net proceeds of at least \$75.0 million for Series A and \$50.0 million for Series B and at an offering price per share greater than or equal to \$4.30 per share for Series A and \$3.66 per share for Series B or upon the decision of the holders of at least a majority of the outstanding

preferred stock shares. Prior to the Mandatory Conversion Time (as defined in the preferred stock purchase agreement), a preferred stockholder would have had the right to elect, upon written notice to the Company, to have all or a portion of its shares of convertible preferred stock automatically convert into shares of Class B common stock at the then effective conversion rate.

### ***Redemption***

The convertible preferred stock was subject to redemption under certain deemed liquidation events not solely within the control of the Company, as defined, and as such was considered contingently redeemable for accounting purposes and was classified as temporary equity in the Company's consolidated balance sheets.

## **13. Stockholders' Equity (Deficit)**

### ***Common stock and pre-funded warrants***

The Company is authorized to issue 450,000,000 shares of common stock with a par value of \$0.000001 per share as of December 31, 2023.

At the effective time of the Reverse Merger on December 18, 2023, the Company issued an aggregate number of 10,472,635 shares of Company common stock to the Neurogene OpCo stockholders based on the Exchange Ratio, including those shares of Neurogene OpCo common stock issued upon the conversion of Neurogene OpCo preferred stock and those shares of the Neurogene OpCo Class A common stock issued in the Pre-Closing Financing, resulting in 12,823,665 shares of Company common stock being issued and outstanding immediately following the effective time of the Reverse Merger.

The Company has pre-funded warrants outstanding to purchase an aggregate of 4,063,361 shares of common stock as of December 31, 2023. The pre-funded warrants are exercisable at any time for an exercise price of \$0.000001, except that the pre-funded warrants cannot be exercised by the holders if, after giving effect thereto, the holders would beneficially own more than 9.99% of the outstanding common stock, subject to certain exceptions. However, any holder may increase or decrease such percentage to any other percentage (not in excess of 19.99%) upon at least 61 days' prior notice from the holder to the Company. The holders of the pre-funded warrants will not have the right to vote the shares underlying the pre-funded warrants on any matter except to the extent required by Delaware law. These warrants were classified as equity.

### ***Class A and Class B Common stock***

Prior to the effective time of the reverse merger, Class A and Class B common stock had the followings rights and privileges:

The holders of Neurogene OpCo Class A common stock were entitled to one vote for each share of Class A common stock held at all meetings of stockholders. Unless required by law, there was no cumulative voting. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company, after the payment of all preferential amounts required to be paid to the holders of shares of preferred stock, the remaining funds and assets available for distribution to the stockholders of the Company would have been distributed among the holders of shares of Class A and Class B common stock, pro rata basis based on the number of shares of Class A and Class B common stock held by each such holder.

Holders of shares of Class B common stock had rights to convert each share of Class B common stock held into one share of Class A common stock at their election, provided that, following the closing of any initial public offering (as defined above), the shares of the Class B common stock would have been converted into shares of Class A common stock unless such conversion would have resulted in a holder owning in excess of 4.99% of any class of securities.

Neurogene OpCo had reserved shares of common stock for future issuance as follows:

	December 31,	
	2023	2022
Conversion of Series A-1 <sup>(1)</sup>	—	18,604,653
Conversion of Series A-2 <sup>(1)</sup>	—	13,291,208
Conversion of Series B <sup>(1)</sup>	—	74,405,719
Total conversion of preferred stock	—	106,301,580
Options outstanding	823,833	468,632
Shares available for future grant under the 2018 Equity Incentive Plan	—	315,548
Shares available for future grant under the 2023 Equity Incentive Plan	2,237,722	—
Total common stock reserved	3,061,555	107,085,760

(1) The convertible preferred stock does not reflect the application of the 0.0756 Exchange Ratio.

#### 14. Stock-Based Compensation

In connection with the reverse merger, the Company stockholders approved the 2023 Equity Incentive Plan (the “2023 EIP”) on December 13, 2023 and Board of Directors ratified the 2023 EIP on December 18, 2023. The 2023 EIP provides for the grant of stock options, restricted stock, restricted stock units (“RSUs”) and other stock-based awards, any of which may be performance-based, and for incentive bonuses, which may be paid in cash, Company common stock or a combination thereof.

The number of shares reserved for issuance under the 2023 EIP is equal to 2,237,722 shares of the Company’s common stock. The 2023 EIP provides that the number of shares reserved and available for issuance under the 2023 EIP will automatically increase on January 1 of each year beginning in 2024 and ending with a final increase on January 1, 2033 in an amount equal to 4% of the total number of shares of common stock outstanding on such date or to a lesser amount determined by the Compensation Committee of the Board of Directors.

As of December 31, 2023, no shares of the Company’s common stock were issued under the 2023 EIP.

In connection with the reverse merger, the Company assumed all of the options outstanding under the Neurogene OpCo 2018 Equity Incentive Plan. All of the stock options outstanding under the 2018 Stock Incentive Plan at the time of the Closing of the reverse merger were adjusted to the number of shares and exercise price to reflect the Exchange Ratio. As of December 31, 2023, 609,124 shares of the Company’s common stock were outstanding under the Neurogene OpCo 2018 Equity Incentive Plan and no further grants will be made under the Neurogene OpCo 2018 Equity Incentive Plan.

In connection with the reverse merger, the Company assumed all of the options outstanding under the Neoleukin 2014 Equity Incentive Plan. As of December 31, 2023, 214,709 shares of the Company’s common stock were outstanding under the Neoleukin 2014 Equity Incentive Plan and no further grants will be made under the Neoleukin 2014 Equity Incentive Plan.

The Company measures stock-based awards at their grant-date fair value and records compensation expense on a straight-line basis over the vesting period of the awards. The Company recorded stock-based compensation expense in the following expense categories in its accompanying consolidated statements of operations (in thousands):

	Year Ended December 31,	
	2023	2022
Research and development	\$ 897	\$ 732
General and administrative	521	520
Total expense	\$ 1,418	\$ 1,252

The following table summarizes the option activity under the stock option plans:

	Number of shares	Weighted average exercise price per share	Weighted average remaining contractual term (years)
Outstanding at December 31, 2022	468,632	\$ 13.23	7.37
Assumption of options in connection with the reverse merger	215,207	\$ 77.20	2.50
Granted	183,391	\$ 18.91	
Exercised	(20,348)	\$ 8.24	
Expired/Forfeited	(23,049)	\$ 20.34	
Outstanding at December 31, 2023	823,833	\$ 31.43	5.89
Exercisable at December 31, 2023	527,053	\$ 38.42	4.73

At December 31, 2023, the aggregate intrinsic value of outstanding options and exercisable options was approximately \$2.6 million and \$2.3 million, respectively. The aggregate intrinsic value of options exercised was approximately \$0.3 million for the year ended December 31, 2023.

The weighted-average grant date fair value of options granted was \$12.99 and \$15.37 per share for the years ended December 31, 2023 and 2022, respectively. The Company recorded stock-based compensation related to stock options of approximately \$1.4 million and \$1.0 million for the years ended December 31, 2023 and 2022, respectively. As of December 31, 2023, the total unrecognized compensation expense related to unvested stock option awards was approximately \$3.2 million, which the Company expects to recognize over a weighted-average period of 2.59 years.

The following table summarizes information about the outstanding and exercisable options at December 31, 2023 (in thousands, except share and per share amounts):

Exercise Price Range	Options Outstanding				Options Exercisable			
	Number Outstanding	Weighted Average Remaining Contractual	Weighted Average Exercise Price	Intrinsic Value	Number Exercisable	Weighted Average Remaining Contractual	Weighted average Exercise Price	Intrinsic Value
\$5.19 - \$9.99	108,600	5.12	\$ 5.86	\$ 1,469	108,315	5.12	\$ 5.86	\$ 1,465
\$10.00 - \$19.99	365,112	6.95	\$ 15.06	\$ 1,596	184,871	5.37	\$ 13.42	\$ 1,120
\$20.00 - \$29.99	196,723	7.14	\$ 22.43	\$ —	80,817	7.41	\$ 22.88	\$ —
\$30.00 - \$274.80	153,398	2.27	\$ 100.05	\$ —	153,050	2.26	\$ 99.86	\$ —

The fair value of each option was estimated on the date of grant using the weighted average assumptions in the table below:

	Year Ended December 31,	
	2023	2022
Expected volatility	82.96%-83.70%	71.71%-74.01%
Risk-free interest rate	3.45%-4.46%	1.47%-4.22%
Expected life (in years)	3.58-6.08	5.87-6.09
Expected dividend yield	—	—
Fair value of common stock	\$ 18.39	\$ 20.24

**Restricted Stock Awards**

The Company granted shares of its restricted Class A common stock to certain of its employees in accordance with the terms of their restricted stock award agreements (“RSA”).

The following table summarizes restricted Class A common stock activity:

	Number of shares	Weighted- average grant date fair value
Unvested balance at December 31, 2022	6,691	\$ 5.82
Vested	(6,691)	\$ 5.82
Unvested balance at December 31, 2023	—	\$ —

The Company recorded stock-based compensation expense related to RSAs of approximately \$0.02 million and \$0.2 million for the years ended December 31, 2023, and 2022, respectively.

**Employee Stock Purchase Plan**

In connection with the reverse merger, the Company stockholders approved the 2023 Employee Stock Purchase Plan (the “2023 ESPP”) on December 13, 2018 and Board of Directors ratified the 2023 ESPP on December 18, 2018. A total of 173,223 shares of common stock have been reserved for issuance under the 2023 ESPP.

Subject to share and dollar limits as described in the plan, the 2023 ESPP allows eligible employees to contribute, through payroll deductions, up to 15% of their earnings for the purchase of the Company’s shares of common stock at the lower of 85% of the closing price of the Company’s common stock on the first trading day of the offering period or 85% of the closing price of the Company’s common stock on the last trading day of the offering period. As of December 31, 2023, no shares have been issued under the 2023 ESPP.

**15. Income Taxes**

Income (loss) before provision for income taxes consisted of the following (in thousands):

	Year Ended December 31,	
	2023	2022
United States	\$ (36,317)	\$ (55,189)
Loss before provision for income taxes	\$ (36,317)	\$ (55,189)

No provision for federal or state income taxes was recorded during the years ended December 31, 2023 and 2022, as the Company incurred operating losses and maintains a full valuation allowance against its net deferred tax assets. The reported amount of income tax benefit for the years ended December 31, 2023 and 2022 differs from the amount that would result from applying domestic federal statutory rates to pretax losses primarily because of changes in the valuation allowance, state taxes, and the generation of research and development credits.



The reconciliation of the Federal statutory income tax provision to the Company's effective income tax provision is as follows:

	Year Ended December 31,	
	2023	2022
Federal statutory income tax	21.0 %	21.0 %
State income taxes, net of federal tax benefit	(0.8)%	(0.1 %)
Other permanent items	(0.5)%	(0.4) %
Research and development credit	5.8 %	3.0 %
Bargain purchase gain	9.5 %	— %
Valuation allowance	(35.0)%	(23.5) %
Effective income tax rate	—	—

Significant components of the Company's net deferred tax assets are as follows (in thousands):

	Year Ended December 31,	
	2023	2022
Deferred tax assets:		
Net operating loss carryforwards	\$ 60,287	\$ 25,253
Research and development credits	9,765	3,681
Accruals & reserves	802	727
Stock compensation	2,952	107
Amortization	778	298
Lease liability	3,157	995
Capitalized research and development expenses	22,134	8,271
Total deferred tax assets	<u>99,875</u>	<u>39,332</u>
Valuation allowance	(98,926)	(38,168)
Net deferred tax assets	<u>949</u>	<u>1,164</u>
Deferred tax liabilities:		
Depreciation	(152)	(224)
Right of use asset	(797)	(940)
Total deferred tax liabilities	<u>(949)</u>	<u>(1,164)</u>
Net deferred taxes	<u>\$ —</u>	<u>\$ —</u>

As of December 31, 2023 and 2022, the Company had a federal net operating loss carryforward of \$277.9 million and \$110.5 million, respectively, which may be available to offset future income tax liabilities. Of this amount, approximately \$2.3 million will begin to expire in 2038 and approximately \$275.6 million are carried forward indefinitely. As of December 31, 2023 and 2022, the Company has state NOL carryforwards of \$35.1 million and \$36.0 million, respectively. Of this amount, approximately \$33.4 million will begin to expire in 2038 and approximately \$1.7 million are carried forward indefinitely.

As of December 31, 2023 and 2022, the Company has federal research and development tax credit carryforwards of \$7.5 million and \$2.9 million, respectively, which begin to expire in 2039. As of December 31, 2023, the Company has federal orphan drug tax credit carryforwards of \$2.2 million, which begin to expire in 2042. As of December 31, 2023, the Company has state research and development tax credit carryforwards of \$0.1 million which begin to expire in 2036.

Future realization of the tax benefits of existing temporary differences and net operating loss carryforwards ultimately depends on the existence of sufficient taxable income within the carryforward period. As of December 31, 2023 and 2022, the Company performed an evaluation to determine whether a valuation allowance was needed. The Company considered all available evidence, both positive and negative, which included the results of operations for the current and preceding years. The Company determined that it was not possible to reasonably quantify future taxable income and determined that it is more likely than not that all of the deferred tax assets will not be realized. Accordingly, the Company maintained a full valuation allowance as of December 31, 2023 and 2022.

The Tax Cuts and Jobs Act (TCJA) resulted in significant changes to the treatment of research and developmental (R&D) expenditures under Section 174 of the IRC. For tax years beginning after December 31, 2021, taxpayers are required to capitalize and amortize all R&D expenditures that are paid or incurred in connection with their trade or business. Specifically, costs for U.S.-based R&D activities must be amortized over five years and costs for foreign R&D activities must be amortized over 15 years—both using a midyear convention. As of December 31, 2023, the Company capitalized a substantial amount of R&D expenditures primarily related to research and development activities performed in the US.

The Company's valuation allowance increased by \$60.7 million and \$13.0 million for the years ended December 31, 2023 and 2022, respectively, due primarily to the generation of NOLs. The Company's valuation allowance for the years ended December 31, 2023 and 2022 is as follows (in thousands):

	Year Ended December 31,	
	2023	2022
Valuation allowance beginning of year	\$ 38,168	\$ 25,195
Increases recorded to income tax provision	12,701	12,973
Increase recorded to equity	48,057	—
Valuation allowance at end of year	<u>\$ 98,926</u>	<u>\$ 38,168</u>

Utilization of the net operating loss and research and development credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986 due to ownership change limitations that have occurred previously or that could occur in the future. These ownership changes may limit the amount of net operating loss and research and development credit carryforwards that can be utilized annually to offset future taxable income and tax, respectively. The Company has not completed a study to assess whether a change of ownership has occurred, or whether there have been multiple ownership changes since its formation, due to the significant cost and complexity associated with a study. There could also be additional ownership changes in the future which may result in additional limitations on the utilization of net operating loss carryforwards and tax credits.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending tax examinations. The Company's tax returns are open under statute from 2018 to the present.

As of December 31, 2023 and 2022, the Company had liabilities for uncertain tax positions of \$2.6 million and \$1.1 million, respectively, which, if recognized, would impact the Company's effective income tax rate. The Company's policy is to record interest and penalties related to income taxes as part of its income tax provision. As of December 31, 2023 and 2022, the Company had not accrued interest or penalties related to uncertain tax positions.

A reconciliation of the beginning and ending amount of unrecognized tax benefits is as follows (in thousands):

	Year Ended December 31,	
	2023	2022
Beginning balance	\$ 1,108	\$ 632
Additions based on tax positions related to current year	574	476
Additions for tax positions of prior years	929	—
Ending Balance	<u>\$ 2,611</u>	<u>\$ 1,108</u>

#### 16. Employee Benefit Plan

The Company sponsors a 401(k) Plan. Employees become eligible for participation upon the start of employment. Participants may elect to have a portion of their salary deferred and contributed to the 401(k) Plan up to the limit allowed under the Internal Revenue Code. The Company makes a matching contribution to the plan for each participant who has elected to make tax-deferred contributions for the plan year. The Company made matching contributions which amounted to approximately \$0.5 million and \$0.5 million for each of the years ended December 31, 2023 and 2022, respectively. These amounts were charged to the consolidated statement of operations. The employer contributions vest over a five-year period.

#### 17. Employee Retention Credit

Pursuant to the Coronavirus Aid, Relief, and Economic Security Act, the Company is eligible for an employee retention credit subject to certain criteria. Since there are no generally accepted accounting principles for for-profit business entities that receive government assistance that is not in the form of a loan, an income tax credit or revenue from a contract with a customer, the Company determined the appropriate accounting treatment by analogy to other guidance. The Company accounted for the employee retention credit by recognizing the employee retention credit on a systematic basis over the periods in which the entity recognizes the payroll expenses for which the grant (i.e., tax credit) is intended to compensate when there is reasonable assurance (i.e., it is probable) that the entity will comply with any conditions attached to the grant and the grant (i.e., tax credit) will be received.

The Company accounted for and received approximately \$0.9 million of employee retention credits during the years ended December 31, 2023, which was recorded as a reduction of research and development expenses and general and administrative expenses on the consolidated statement of operations.

#### 18. Subsequent Events

From January 1, 2024 until the financial statements were issued, the Company granted 740,752 options and 482,384 RSUs to employees and consultants, and 29,320 options were exercised.

**Neurogene Inc.**  
**Condensed Consolidated Balance Sheets**  
(In Thousands, Except Share Information)  
(Unaudited)

	September 30, 2024	December 31, 2023
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 66,633	\$ 148,210
Short-term investments	72,360	48,947
Prepaid expenses and other current assets	4,654	3,191
Total current assets	143,647	200,348
Property and equipment, net	16,068	17,174
Operating lease right-of-use assets	3,193	3,681
Finance lease right-of-use assets	84	98
Restricted cash	339	508
Other non-current assets	789	764
Total assets	\$ 164,120	\$ 222,573
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 1,684	\$ 2,596
Accrued expenses and other current liabilities	10,438	17,495
Operating lease liabilities, current	2,856	2,559
Finance lease liabilities, current	54	42
Contingent value rights liability, current	768	281
Total current liabilities	15,800	22,973
Operating lease liabilities, non-current	10,170	12,302
Finance lease liabilities, non-current	39	65
Contingent value rights liability, non-current	646	1,006
Other liabilities	51	203
Total liabilities	26,706	36,549
Stockholders' equity:		
Preferred stock, \$0.000001 par value; 50,000,000 shares authorized as of September 30, 2024 and December 31, 2023; 0 shares issued and outstanding as of September 30, 2024 and December 31, 2023	—	—
Common stock, \$0.000001 par value; 450,000,000 shares authorized as of September 30, 2024 and December 31, 2023; 13,010,867 and 12,823,665 shares issued and outstanding as of September 30, 2024 and December 31, 2023, respectively	—	—
Additional paid-in capital	380,198	373,178
Accumulated deficit	(242,784)	(187,154)
Total stockholders' equity	137,414	186,024
Total liabilities and stockholders' equity	\$ 164,120	\$ 222,573

The accompanying notes are an integral part of these condensed consolidated financial statements.

**Neurogene Inc.**  
**Condensed Consolidated Statements of Operations**  
(In Thousands, Except Share Information)  
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Revenue under licensing agreements	\$ —	\$ —	\$ 925	\$ —
Operating expenses:				
Research and development expenses	16,285	11,606	45,570	32,210
General and administrative expenses	5,895	3,613	16,448	8,640
Total operating expenses	22,180	15,219	62,018	40,850
Loss from operations	(22,180)	(15,219)	(61,093)	(40,850)
Other income (expense):				
Interest income	1,842	650	6,197	2,170
Interest expense	(3)	(4)	(10)	(9)
Other income	143	—	430	—
Other expense	(19)	(4)	(1,154)	(11)
Net loss	\$ (20,217)	\$ (14,577)	\$ (55,630)	\$ (38,700)
Per share information: <sup>(1)</sup>				
Net loss per share, basic and diluted	\$ (1.19)	\$ (32.67)	\$ (3.29)	\$ (87.66)
Weighted-average shares of common stock outstanding, basic and diluted	16,953,443	446,255	16,932,976	441,498

(1) For the three and nine months ended September 30, 2023, net loss per share information is presented for the Company's then outstanding Class A common stock. For the three and nine months ended September 30, 2024, net loss per share information is presented for the Company's common stock. See Note 1, *Reverse Merger and Pre-Closing Financing* and Note 3, *Net Loss Per Share Attributable to Common Stockholders*, for additional information.

*The accompanying notes are an integral part of these condensed consolidated financial statements.*

**Neurogene Inc.**  
**Condensed Consolidated Statements of Changes in Convertible Preferred Stock and Stockholders' Equity (Deficit)**  
**(In Thousands, Except Share Information)**  
**(Unaudited)**

	Convertible Preferred Stock						Stockholders' Equity										
	Series A-1 Convertible Preferred Stock		Series A-2 Convertible Preferred Stock		Series B Convertible Preferred Stock		Preferred Stock		Class A Common Stock		Class B Common Stock		Common Stock		Additional Paid- In Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount			
<b>Balance- December 31, 2023</b>	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	12,823,665	\$ —	\$ 373,178	\$ (187,154)	\$ 186,024
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	—	—	1,045	—	1,045
Common stock issued upon exercise of stock options	—	—	—	—	—	—	—	—	—	—	—	—	37,330	—	629	—	629
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(16,921)	(16,921)
<b>Balance- March 31 2024</b>	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	12,860,995	\$ —	\$ 374,852	\$ (204,075)	\$ 170,777
Shares issued upon the exercise of pre-funded warrants	—	—	—	—	—	—	—	—	—	—	—	—	103,407	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	—	—	2,314	—	2,314
Common stock issued upon exercise of stock options	—	—	—	—	—	—	—	—	—	—	—	—	24,806	—	415	—	415
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(18,492)	(18,492)
<b>Balance- June 30, 2024</b>	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	12,989,208	\$ —	\$ 377,581	\$ (222,567)	\$ 155,014
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	—	—	2,474	—	2,474
Common stock issued upon exercise of stock options	—	—	—	—	—	—	—	—	—	—	—	—	21,659	—	143	—	143
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(20,217)	(20,217)
<b>Balance- September 30, 2024</b>	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	13,010,867	\$ —	\$ 380,198	\$ (242,784)	\$ 137,414

*The accompanying notes are an integral part of these condensed consolidated financial statements.*

**Neurogene Inc.**  
**Condensed Consolidated Statements of Changes in Convertible Preferred Stock and Stockholders' Equity (Deficit)**  
(In Thousands, Except Share Information)  
(Unaudited)

	Convertible Preferred Stock						Stockholders' Deficit										
	Series A-1 Convertible Preferred Stock		Series A-2 Convertible Preferred Stock		Series B Convertible Preferred Stock		Preferred Stock		Class A Common Stock		Class B Common Stock		Common Stock		Additional Paid- In Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount			
<b>Balance-December 31, 2022</b>	18,604,653	\$34,414	13,291,208	\$28,675	74,405,719	\$181,277	—	\$ —	428,334	\$ —	—	\$ —	—	\$ —	\$ 5,098	\$ (150,837)	\$ (145,739)
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	—	—	293	—	293
Class A common stock issued upon exercise of stock options	—	—	—	—	—	—	—	—	13,532	—	—	—	—	—	112	—	112
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(12,263)	(12,263)
<b>Balance-March 31, 2023</b>	18,604,653	\$34,414	13,291,208	\$28,675	74,405,719	\$181,277	—	\$ —	441,866	\$ —	—	\$ —	—	\$ —	\$ 5,503	\$ (163,100)	\$ (157,597)
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	—	—	384	—	384
Class A common stock issued upon exercise of stock options	—	—	—	—	—	—	—	—	2,751	—	—	—	—	—	20	—	20
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(11,860)	(11,860)
<b>Balance-June 30, 2023</b>	18,604,653	\$34,414	13,291,208	\$28,675	74,405,719	\$181,277	—	\$ —	444,617	\$ —	—	\$ —	—	\$ —	\$ 5,907	\$ (174,960)	\$ (169,053)
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	—	—	386	—	386
Class A common stock issued upon exercise of stock options	—	—	—	—	—	—	—	—	4,027	—	—	—	—	—	35	—	35
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(14,577)	(14,577)
<b>Balance-September 30, 2023</b>	18,604,653	\$34,414	13,291,208	\$28,675	74,405,719	\$181,277	—	\$ —	448,644	\$ —	—	\$ —	—	\$ —	\$ 6,328	\$ (189,537)	\$ (183,209)

*The accompanying notes are an integral part of these condensed consolidated financial statements.*

**Neurogene Inc.**  
**Condensed Consolidated Statements of Cash Flows**  
(In Thousands)  
(Unaudited)

	Nine Months Ended September 30,	
	2024	2023
<b>Operating activities</b>		
Net loss	\$ (55,630)	\$ (38,700)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	5,833	1,063
Depreciation and amortization of property and equipment	2,410	2,445
Asset impairment	91	—
Non-cash operating lease expense	548	492
Amortization of finance lease right-of-use assets	39	24
Amortization and accretion of premiums/discounts on held-to-maturity investments	(1,460)	—
Contingent value rights liability	127	—
Changes in assets and liabilities:		
Prepaid expenses and other current assets	(1,454)	(870)
Other assets	(25)	—
Accounts payable	339	1,509
Accrued expenses and other liabilities	(2,030)	88
Operating lease liabilities	(1,895)	(501)
Net cash used in operating activities	(53,107)	(34,450)
<b>Investing activities</b>		
Purchases of property and equipment	(692)	(193)
Purchases of held-to-maturity investments	(80,203)	—
Proceeds from maturities of held-to-maturity investments	58,250	—
Net cash used in investing activities	(22,645)	(193)
<b>Financing activities</b>		
Offering costs in connection with pre-closing financing	(4,287)	(1,961)
Transaction costs related to reverse merger	(2,855)	—
Proceeds from the issuance of common stock upon exercise of options	1,187	167
Principal payments on finance leases	(39)	(21)
Net cash used in financing activities	(5,994)	(1,815)
Net decrease in cash, cash equivalents and restricted cash	(81,746)	(36,458)
Cash, cash equivalents and restricted cash at beginning of period	148,718	82,021
Cash, cash equivalents and restricted cash at end of period	\$ 66,972	\$ 45,563
<b>Supplemental disclosure of non-cash investing and financing activities:</b>		
Additions to operating lease right of use assets from new operating lease liabilities	\$ 60	\$ —
Property and equipment included in accounts payable and accrued expenses	\$ 703	\$ —
Additions to finance lease right of use assets from new finance lease liabilities	\$ 25	\$ 46
Deferred offering costs in accrued expenses	\$ 9	\$ 1,095
<b>Supplemental cash flow information:</b>		
Cash paid for interest	\$ 10	\$ 9

*The accompanying notes are an integral part of these condensed consolidated financial statements.*



**NEUROGENE INC.****NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (UNAUDITED)**

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**1. Organization and Description of Business**

Neurogene Inc. (formerly known as Neoleukin Therapeutics, Inc.) (the “Company” or “Neurogene”) is a clinical-stage biotechnology company that is a result of the reverse merger discussed below. The operating entity of Neurogene Inc. is the wholly owned subsidiary incorporated in the state of Nevada and also named Neurogene Inc. (“Neurogene OpCo”). Neurogene OpCo was incorporated as a limited liability company in Delaware on January 26, 2018 and converted into a Delaware corporation on July 3, 2018, and then merged with a wholly owned subsidiary of the parent company and re-domiciled to Nevada on December 18, 2023 in connection with the reverse merger described below. Both Neurogene and Neurogene OpCo have a principal place of business in New York, NY. Neurogene was formed to harness the power of gene therapy, combined with its EXACT™ transgene regulation technology, to turn today’s complex devastating neurological diseases into treatable conditions. The Company’s first clinical-stage program to utilize the EXACT technology is NGN-401, which is in an ongoing Phase 1/2 clinical trial for the treatment of Rett syndrome. Since beginning operations, the Company has devoted substantially all its efforts to research and development, recruiting management and technical staff, administration, and raising capital.

***Reverse Merger and Pre-Closing Financing***

On July 18, 2023, Neoleukin Therapeutics, Inc. entered into an Agreement and Plan of Merger (the “Merger Agreement”) with its wholly owned subsidiary (“Merger Sub”) and Neurogene OpCo. Pursuant to the terms of the Merger Agreement, upon closing on December 18, 2023 (the “Closing”), Merger Sub merged with and into Neurogene OpCo, with Neurogene OpCo continuing as a wholly owned subsidiary of the Company and the surviving corporation of the merger (referred to herein as the “reverse merger”).

At the time of Closing (or immediately prior to, where indicated), the following also occurred:

- The Company changed its name from “Neoleukin Therapeutics, Inc.” to “Neurogene Inc.” and is referred to herein as the “Company.” Unless the context otherwise requires, references to “Neoleukin Therapeutics, Inc.” or “Neoleukin” refer to the Company prior to Closing.
- Immediately prior to Closing, Neoleukin effected a 1-for-4 reverse stock split (the “Reverse Stock Split”). Unless noted otherwise, all references herein to share and per share amounts reflect the Reverse Stock Split.
- All of the then outstanding shares of Neurogene OpCo Class A common stock were converted into 3,240,888 shares of the Company’s common stock, based on an exchange ratio of approximately 0.0756 (the “Exchange Ratio”).
- All of the then outstanding shares of Neurogene OpCo preferred stock were converted into 7,231,747 shares of the Company’s common stock and 1,825,635 pre-funded warrants, based on the Exchange Ratio.
- Each then outstanding Neurogene OpCo stock option was exchanged for an equivalent stock option of the Company, adjusted to reflect the Exchange Ratio as necessary.
- Each then outstanding Neurogene OpCo pre-funded warrant to purchase shares of Neurogene OpCo common stock was converted into a pre-funded warrant to purchase shares of the Company’s common stock, adjusted to reflect the Exchange Ratio as necessary. Refer to the discussion below for further detail on Neurogene OpCo pre-funded warrants.

Concurrently with the execution and delivery of the Merger Agreement, and in order to provide Neurogene OpCo with additional capital for its development programs, Neurogene OpCo entered into a subscription agreement (the “Subscription Agreement”) with certain investors. Pursuant to the terms of the Subscription Agreement, immediately prior to the Closing, Neurogene OpCo issued and sold to the investors: (i) 2,792,206 shares of Neurogene OpCo common stock and (ii) 1,811,739 pre-funded warrants, exercisable for 1,811,739 shares of

Neurogene OpCo common stock, at a purchase price of approximately \$20.63 per share or \$20.63 per warrant, for an aggregate purchase price of approximately \$95.0 million (the “Pre-Closing Financing”).

## **2. Risks and Uncertainties**

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, successful development of technology, obtaining additional funding, protection of proprietary technology, compliance with government regulations, risks of failure of pre-clinical studies, clinical studies and clinical trials, the need to obtain marketing approval for its drug candidates and its consumer products, fluctuations in operating results, economic pressure impacting therapeutic pricing and reimbursement, dependence on key personnel, risks associated with changes in technologies, development by competitors of technological innovations and the ability to transition from pilot scale manufacturing to large scale production.

### ***Liquidity and Financial Condition***

Since its inception, the Company has funded its operations primarily with proceeds from the sales of equity securities and has incurred significant recurring losses, including net losses of \$55.6 million and \$38.7 million for the nine months ended September 30, 2024 and 2023, respectively. In addition, the Company used cash in operations of \$53.1 million and \$34.5 million for the nine months ended September 30, 2024 and 2023, respectively, and had an accumulated deficit of \$242.8 million as of September 30, 2024. Management expects to incur substantial and increasing losses in future periods as the Company advances its products through its clinical and regulatory process and will rely on outside capital to fund its operations for the foreseeable future. The Company has not generated positive cash flows from operations, and there are no assurances that the Company will be successful in obtaining an adequate level of financing for the development and commercialization of its product candidates.

As of September 30, 2024, the Company had cash, cash equivalents and investments of approximately \$139.0 million. On December 18, 2023, the Company closed the reverse merger and the Pre-Closing Financing. The Company expects its available cash and cash equivalents on hand as of the issuance date of these financial statements will be sufficient to fund its obligations as they become due for at least one year beyond the issuance date of these financial statements. As a result of the reverse merger with Neoleukin Therapeutics, the Company assumed an ATM or “at-the-market” Equity Offering Sales Agreement with BofA Securities, Inc., as agent (“BofA”), pursuant to which the Company was able to offer and sell, from time to time through BofA, shares of the Company’s common stock, having an aggregate offering price of up to \$40.0 million. The Registration Statement on Form S-3 that was prepared for the ATM expired on December 21, 2023, and in March 2024, the Company formally terminated the ATM.

In the event the Company is unable to secure additional outside capital, management will be required to seek other alternatives which may include, among others, a delay or termination of clinical trials or the development of its product candidates, temporary or permanent curtailment of the Company’s operations, a sale of assets, or other alternatives with strategic or financial partners.

The accompanying condensed consolidated financial statements do not include any adjustments that might result from the outcome of these uncertainties. Accordingly, the condensed consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

## **3. Summary of Significant Accounting Policies**

### ***Basis of Presentation***

The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America (“U.S. GAAP”) and pursuant to the rules and regulations of the United States Securities and Exchange Commission (“SEC”) for interim financial information. Accordingly, these financial statements do not include all of the information and footnotes required by U.S. GAAP for complete financial statements and should be read in conjunction with the audited consolidated

financial statements and accompanying notes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023, filed with the SEC on March 18, 2024.

In management's opinion, the unaudited condensed consolidated financial statements reflect all adjustments (consisting of normal recurring adjustments) necessary to present fairly the financial position of the Company as of September 30, 2024, and results of operations and cash flows for all periods presented. The interim results presented are not necessarily indicative of results that can be expected for the full year ending December 31, 2024.

### ***Use of Estimates***

The preparation of the financial statements in accordance with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenue and expenses during the reporting period. In preparing these financial statements, management used significant estimates in the following areas, among others: recoverability of the Company's net deferred tax assets and related valuation allowance, useful lives and recoverability of property and equipment, determining the incremental borrowing rate for calculating lease liabilities and related right-of-use assets and finance lease assets, revenue recognition, clinical trial accruals, accrual estimates for all contingent value rights ("CVRs"), the value attributed to employee stock options and other stock-based awards, and valuation of common stock. On an ongoing basis, the Company reviews its estimates to ensure that they appropriately reflect changes in the business or as new information becomes available. Actual results may differ from these estimates.

### ***Segment Information***

Operating segments are defined as components of an entity for which separate financial information is available and that is regularly reviewed by the Chief Operating Decision Maker ("CODM") in deciding how to allocate resources and in assessing performance. The Company's CODM is the Chief Executive Officer. The CODM assesses performance of the segment according to clinical and pre-clinical data along with program specific expenses and market conditions in the pharmaceutical and biotechnology sectors. The Company operates as a single operating segment and has one reportable segment. The Company's operations and its assets are held in the United States.

### ***Cash and Cash Equivalents***

The Company considers all highly-liquid investments purchased with original maturities of 90 days or less at time of purchase to be cash equivalents. Cash and cash equivalents include cash held in banks and are stated at fair value.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash in the balance sheets that sum to the total of the same such amounts shown in the statements of cash flows (in thousands):

	September 30, 2024	December 31, 2023
Cash and cash equivalents	\$ 66,633	\$ 148,210
Restricted cash	339	508
Total cash, cash equivalents and restricted cash	<u>\$ 66,972</u>	<u>\$ 148,718</u>

Cash equivalents consist of money market funds in which the carrying value equals the fair value. Restricted cash includes \$0.3 million in cash deposits the Company maintains with its bank as collateral for the irrevocable letters of credits related to its lease obligations.

### ***Concentrations of Credit Risk***

Financial instruments that subject the Company to significant concentrations of credit risk consist primarily of cash and cash equivalents. The Company's cash and cash equivalent accounts, at times, may exceed federally

insured limits. As of September 30, 2024, the Company had \$66.5 million in excess of the federally insured limits. The Company places its cash in financial institutions that management believes to be of high credit quality.

### ***Revenue Recognition***

The Company recognizes revenue when its customers obtain control of promised goods or services in an amount that reflects the consideration that the Company expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements within the scope of Accounting Standards Codification (“ASC”) 606, Revenue from Contracts with Customers, (“ASC 606”) the Company performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the performance obligation is satisfied.

In applying the ASC 606 framework, the Company must apply judgment to determine the nature of the promises within a revenue contract and whether those promises represent distinct performance obligations. In determining the transaction price, the Company does not include amounts subject to uncertainties unless it is probable that there will be no significant reversal of cumulative revenue when the uncertainty is resolved. Milestone and other forms of variable consideration that the Company may earn are subject to significant uncertainties of research and development related achievements, which generally are deemed not probable until such milestones are actually achieved. For arrangements that include sales-based royalties, including milestone payments based on the level of sales, and where the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). Additionally, the Company develops assumptions that require judgment to determine the standalone selling price of each performance obligation identified in the contract. The Company then allocates the total transaction price to each performance obligation based on the estimated standalone selling prices of each performance obligation for which it recognizes revenue as or when the performance obligations are satisfied. At the end of each subsequent reporting period, the Company re-evaluates the variable consideration and any related constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis.

Under the Company’s license agreements, the Company grants the license to a customer as it exists at the point of transfer and the nature of the license is a right to use the Company’s intellectual property as transferred. If the license to the Company’s intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. As of September 30, 2024, the Company has two revenue-generating agreements that are related to the legacy Neoleukin business as part of the reverse merger: the December 2023 CVR Licensing Agreement (as defined below) and the April 2024 CVR Licensing Agreement (as defined below). Refer to Note 9, Commitments and Contingencies, for further discussion on the CVR components.

### ***Contingent Value Rights***

In conjunction with the reverse merger, the Company entered into a CVR Agreement on December 18, 2023 with the Rights Agent named therein (the “CVR Agreement”) prior to Closing. Included in the CVR Agreement are three different types of CVRs: (i) the Lease CVR, (ii) the Intellectual Property CVR, and (iii) the Sales Tax CVR (each as defined in the CVR agreement). The Company evaluated each of the CVRs to determine if they qualified as derivatives under ASC 815, Derivatives and Hedging, and concluded that since certain scope exceptions were met, the CVRs did not qualify as derivatives. Instead, the Company records a contingent consideration liability associated with the CVRs when payments are probable and estimable under ASC 450, Contingencies. In assessing whether payments are probable and estimable, the Company considers the existence of or ability to enter into agreements with third parties or government agencies and the timing of potential payments. Refer to Note 9, Commitments and Contingencies, for further discussion on the CVRs.

### ***Exit and Disposal Costs***

In connection with the reverse merger and through early fiscal 2025, the Company has incurred and expects to incur costs to wind-down Neoleukin's Phase 1 trial of NL-201. This trial has ceased further development, and the Company has no plans to continue developing Neoleukin's *de novo* protein technology. As a result, the trial's activities do not provide the Company any future economic benefit. In accordance with ASC 420, *Exit or Disposal Costs*, the Company accrued the remaining costs to be incurred in the trial. The liability was classified as accrued expenses and other current liabilities in the condensed consolidated balance sheet.

A summary of the accrued liabilities activity recorded in connection with the wind-down of Neoleukin's Phase 1 trial of NL-201 for the nine months ended September 30, 2024 is as follows (in thousands):

	<b>Balance at December 31, 2023</b>	<b>Liability Adjustment</b>	<b>Amounts Paid</b>	<b>Balance at September 30, 2024</b>
Trial wind-down costs:				
Phase 1 NL-201 Trial	\$ 1,962	\$ (201)	\$ (999)	\$ 762

### ***Significant Accounting Policies***

The Company's significant accounting policies are disclosed in the audited financial statements filed with the Company's Annual Report on Form 10-K for the year ended December 31, 2023. Since the date of those financial statements, there have been no changes to the Company's significant accounting policies.

#### ***Net Loss Per Share Attributable to Common Stockholders***

For the prior year period in which the Company had multiple classes of stock participating in earnings, the Company used the two-class method in calculating net loss per share. The two-class method requires income available to ordinary shareholders for the period to be allocated between ordinary shares and participating securities based upon their respective rights. The Company considered its convertible preferred stock to be participating securities as holders would be entitled to participate in dividends and earnings of the Company. As the holders of the convertible preferred stock have no obligation to fund losses, the two-class method is not applicable during periods with a net loss.

Basic net loss per share of Class A and Class B common stock was computed by dividing net loss attributable to the Company by the weighted-average number of shares of Class A and Class B common stock outstanding during the period. In periods of losses, diluted net loss per share was computed on the same basis as basic net loss per share as the inclusion of any other potential shares outstanding would be anti-dilutive.

Following the Company's reverse merger in December 2023, the Company only has one class of common stock remaining, referred to throughout as "common stock." Basic net loss per share of common stock is computed by dividing net income attributable to the Company by the weighted-average number of shares of common stock outstanding during the period. In periods of losses, diluted net loss per share is computed on the same basis as basic net loss per share as the inclusion of any other potential shares outstanding would be anti-dilutive.

The following table sets forth the computation of basic and diluted net loss per share (in thousands, except share amounts):

	Nine Months Ended September 30,		
	2024	2023	
	Common stock	Class A	Class B
<b>Numerator:</b>			
Net loss	\$ (55,630)	\$ (38,700)	\$ —
<b>Denominator:</b>			
Weighted-average shares outstanding in computing net loss per share, basic and diluted	16,932,976	441,498	—
<b>Net loss per share, basic and diluted</b>	<b>\$ (3.29)</b>	<b>\$ (87.66)</b>	<b>\$ —</b>

The following potentially dilutive securities have been excluded from the diluted per share calculations as they would be anti-dilutive:

	Nine Months Ended September 30,	
	2024	2023
Series A-1 convertible preferred stock <sup>(1)</sup>	—	18,604,653
Series A-2 convertible preferred stock <sup>(1)</sup>	—	13,291,208
Series B convertible preferred stock <sup>(1)</sup>	—	74,405,719
Stock options	1,384,182	609,656
<b>Total</b>	<b>1,384,182</b>	<b>106,911,236</b>

<sup>(1)</sup> The convertible preferred stock does not reflect the application of the 0.0756 Exchange Ratio.

#### **Recently Issued Accounting Standards**

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board (“FASB”) or other standard setting bodies that the Company adopts as of the specified effective date. Unless otherwise discussed below, the Company does not believe that the adoption of recently issued standards have or may have a material impact on its condensed financial statements or disclosures.

#### **Recently Issued Accounting Pronouncements Not Yet Adopted**

In November 2023, the FASB issued Accounting Standards Update (“ASU”) 2023-07, Segment Reporting (Topic 280). The amendments in this update expand segment disclosure requirements, including new segment disclosure requirements for entities with a single reportable segment among other disclosure requirements. This update is effective for the Company in the consolidated financial statements for the year ending December 31, 2024, and interim periods beginning after January 1, 2025. The Company is analyzing the impact of this standard on its disclosures in the condensed consolidated financial statements.

In December 2023, the FASB issued ASU 2023-09, Improvements to Income Tax Disclosures, which requires consistent categories and greater disaggregation of information in the rate reconciliation and income taxes paid disaggregated by jurisdiction. This standard will be effective for annual periods beginning after December 15, 2024. The Company is currently evaluating the impact of this standard on the Company’s condensed consolidated financial statements and related disclosures.

#### 4. Investments

The following table summarizes the Company's investment securities as of September 30, 2024 and December 31, 2023 (in thousands):

	September 30, 2024			
	Amortized cost, as adjusted	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value
Cash equivalents:				
Money market funds	\$ 63,120	\$ —	\$ —	\$ 63,120
Short-term investments:				
U.S. treasury notes	72,360	172	—	72,532
<b>Total</b>	<b>\$ 135,480</b>	<b>\$ 172</b>	<b>\$ —</b>	<b>\$ 135,652</b>

	December 31, 2023			
	Amortized cost, as adjusted	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value
Cash equivalents:				
Money market funds	\$ 144,358	\$ —	\$ —	\$ 144,358
Short-term investments:				
U.S. treasury notes	48,947	16	—	48,963
<b>Total</b>	<b>\$ 193,305</b>	<b>\$ 16</b>	<b>\$ —</b>	<b>\$ 193,321</b>

All of the Company's investments mature within the next 12 months.

#### 5. Fair Value of Financial Instruments

As of September 30, 2024 and December 31, 2023, financial assets measured at fair value on a recurring basis are categorized in the table below based upon the lowest level of significant input to the valuations (in thousands):

	September 30, 2024		
	Level 1	Level 2	Level 3
Assets:			
Money market funds	\$ 63,120	\$ —	\$ —
U.S. treasury notes	72,532	—	—
<b>Total</b>	<b>\$ 135,652</b>	<b>\$ —</b>	<b>\$ —</b>

	December 31, 2023		
	Level 1	Level 2	Level 3
Assets:			
Money market funds	\$ 144,358	\$ —	\$ —
U.S. treasury notes	48,947	—	—
<b>Total</b>	<b>\$ 193,305</b>	<b>\$ —</b>	<b>\$ —</b>

Money market funds are cash equivalents and are included in cash and cash equivalents in the condensed consolidated balance sheet as of September 30, 2024 and December 31, 2023.

## 6. Prepaid expenses and other current assets

Prepaid expenses and other assets consist of the following (in thousands):

	September 30, 2024	December 31, 2023
Refunds and other receivables	\$ 672	\$ 600
Prepaid expenses	2,382	1,496
Other current assets	1,600	1,095
Total prepaid and other current assets	<u>\$ 4,654</u>	<u>\$ 3,191</u>

## 7. Property and Equipment, Net

Property and equipment, net consist of the following (in thousands):

	September 30, 2024	December 31, 2023
Lab equipment	\$ 3,244	\$ 3,144
Manufacturing equipment	6,326	6,142
Office Equipment	19	19
Leasehold improvements	15,396	15,376
Software	268	268
Construction in progress	1,202	234
Total property and equipment, cost	<u>26,455</u>	<u>25,183</u>
Less accumulated depreciation	<u>(10,387)</u>	<u>(8,009)</u>
Property and equipment, net	<u>\$ 16,068</u>	<u>\$ 17,174</u>

The Company recorded depreciation and amortization expense of \$0.8 million for each of the three months ended September 30, 2024 and 2023. The Company recorded depreciation and amortization expense of \$2.4 million for each of the nine months ended September 30, 2024 and 2023.

Management has reviewed its property and equipment for impairment whenever events and circumstances indicate that the carrying value of an asset might not be recoverable. During the nine months ended September 30, 2024, the Company recorded impairment losses on idle equipment of \$0.1 million, which was charged to research and development expenses in the condensed consolidated statement of operations. Fair value for the idle assets was determined by a quoted purchase price for the assets.

## 8. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consist of the following (in thousands):

	September 30, 2024	December 31, 2023
Compensation, bonuses and related benefits	\$ 3,432	\$ 3,496
Research and development	6,483	4,895
Accrued severance, bonus, and retention <sup>(1)</sup>	—	2,476
Accrued offering costs in connection with pre-closing financing	—	3,334
Accrued transaction costs related to reverse merger	—	2,557
Other	523	737
Total accrued expenses and other current liabilities	<u>\$ 10,438</u>	<u>\$ 17,495</u>

(1) Includes accrued severance, bonus, and retention payments for former Neoleukin employees.



## 9. Commitments and Contingencies

### *Operating and Finance Leases*

Supplemental lease expense related to leases for the three and nine months ended September 30, 2024 and 2023 was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Operating lease cost	\$ 508	\$ 259	\$ 1,553	\$ 777
Finance lease cost				
Amortization of finance leases	12	10	39	24
Interest on finance lease liabilities	3	3	9	9
Variable lease cost	364	19	978	129
Short-term lease cost	18	21	53	63
Total lease cost	\$ 905	\$ 312	\$ 2,632	\$ 1,002

The following table summarizes the maturity of the Company's operating and finance lease liabilities on an undiscounted cash flow basis and a reconciliation to the operating and finance lease liabilities recognized on the Company's condensed consolidated balance sheet as of September 30, 2024 (in thousands):

#### **Maturity of operating lease liabilities**

2024 (remaining)	\$ 982
2025	3,987
2026	3,695
2027	3,239
2028	3,294
2029	613
Total lease payments	\$ 15,810
Less: interest	(2,784)
Total operating lease liabilities	\$ 13,026

#### **Maturity of finance lease liabilities**

2024 (remaining)	\$ 15
2025	60
2026	21
2027	6
Total lease payments	\$ 102
Less: interest	(9)
Total finance lease liabilities	\$ 93

Supplemental balance sheet information related to leases as of September 30, 2024 was as follows (in thousands):

**Leases**

Operating right-of-use assets	\$	3,193
Operating lease liabilities, current		2,856
Operating lease liabilities, non-current		10,170
Total operating lease liabilities	\$	<u>13,026</u>
Finance right-of-use assets	\$	84
Finance lease liabilities, current		54
Finance lease liabilities, non-current		39
Total finance lease liabilities	\$	<u>93</u>

**Other information**

Cash paid for amounts included in measurement of operating lease liabilities (in thousands)	\$	2,899
Cash paid for amounts included in measurement of finance lease liabilities (in thousands)	\$	45
Weighted-average remaining lease term - operating leases (in years)		4.18
Weighted-average remaining lease term - finance lease (in years)		1.87
Weighted-average discount rate - operating leases		9.73 %
Weighted-average discount rate - finance lease		11.05 %

**Lease CVR**

As of September 30, 2024, approximately \$1.2 million was recorded as a component of the contingent value rights liability on the Company's condensed consolidated balance sheet consisting of lease commitments that were probable and estimable at the Closing. The commitments relate to Neoleukin's sublease agreement, effective October 31, 2023, for one of its properties with an unrelated third party for the remainder of the lease term.

**Intellectual Property CVR**

Under the CVR Agreement, each CVR holder is eligible to receive 100% of the net proceeds, if any, derived from any consideration paid as a result of the disposition of Neoleukin's pre-merger legacy assets pursuant any agreements entered into before the Closing, and 80% of net proceeds, if any, derived from any consideration paid as a result of the disposition of Neoleukin's pre-merger legacy assets pursuant any agreements entered into within one year after the Closing (the "Intellectual Property CVR"). Contingent consideration liabilities related to the CVR Agreement will only be recorded if the liabilities are probable and estimable as of the balance sheet date. Refer to the *December 2023 CVR Licensing Agreement and April 2024 CVR Licensing Agreement subsections* below for further detail on the current agreements related to our Intellectual Property CVR.

*December 2023 CVR Licensing Agreement*

Prior to the Closing, Neoleukin entered into a licensing agreement on December 13, 2023 with an unrelated third party to develop and commercialize certain legacy Neoleukin assets (the "December 2023 CVR Licensing Agreement"). In June 2024, an upfront payment of \$0.2 million was received by the Company. Since the December 2023 CVR Licensing Agreement was entered into before the Closing, the CVR holders are eligible to receive 100% of the net proceeds derived from the December 2023 CVR Licensing Agreement. Accordingly, an upfront payment of \$0.2 million was recorded as licensing revenue within the condensed consolidated statements of operations. The December 2023 CVR Licensing Agreement contains development, regulatory and commercialization milestones

totaling up to approximately \$13.4 million, as well as royalty payments. However, as of September 30, 2024, no other development and sales milestones were achieved nor deemed probable of achievement under the December 2023 CVR Licensing Agreement.

#### *April 2024 CVR Licensing Agreement*

In April 2024, the Company entered into a licensing and intellectual property assignment agreement with another unrelated third party to develop and commercialize certain legacy Neoleukin assets (the “April 2024 CVR Licensing Agreement”). In April 2024, the Company received a one-time upfront payment of approximately \$0.8 million and reimbursement of \$10,000 for patent expenses under the April 2024 CVR Licensing Agreement. Since the April 2024 CVR Licensing Agreement was entered into within one year after the Closing, the CVR holders are eligible to receive 80% of the net proceeds derived from the April 2024 CVR Licensing Agreement. Accordingly, the Company has recorded \$0.8 million as licensing revenue within the condensed consolidated statements of operations. The April 2024 CVR Licensing Agreement contains development, regulatory and commercialization milestones totaling up to approximately \$11.0 million, as well as royalty payments. However, as of September 30, 2024, no other development and sales milestones were achieved nor deemed probable of achievement under the April 2024 CVR Licensing Agreement.

The December 2023 CVR Licensing Agreement and April 2024 CVR Licensing Agreement collectively account for the total Intellectual Property CVR. The total amount of \$0.9 million due under the Intellectual Property CVR was offset by approximately \$0.4 million due to deductions permitted under the Merger Agreement and \$0.5 million from the first CVR payment discussed below. As of September 30, 2024, \$0.1 million is recorded within contingent value rights liability as an offset arising from the Intellectual Property CVR on the Company's condensed consolidated balance sheet, which can only be applied against licensing revenues from the December 2023 Licensing Agreement or the April 2024 CVR Licensing Agreement.

#### **Sales Tax CVR**

Prior to the Closing, Neoleukin entered into an agreement with an unrelated third party for refund analysis services of Washington state sales tax. As discussed and defined within the *Contingent Value Rights* section of Note 3, *Summary of Significant Accounting Policies*, the terms of the CVR Agreement include that CVR holders are eligible to receive net proceeds derived from an anticipated sales tax refund from Washington state relating to tax returns filed by Neoleukin prior to Closing. As of September 30, 2024, it was deemed probable that the Company will receive proceeds from Washington state for the sales tax refund and will remit the proceeds to the CVR holders. As of September 30, 2024, \$0.3 million is accrued as a component of the contingent value rights liability arising from the Sales Tax CVR in the condensed consolidated balance sheet.

#### **CVR Payment**

In August 2024, the Company made the first CVR payment to CVR holders, net of expenses, for \$0.6 million. \$0.5 million was applied to the Intellectual Property CVR and \$0.1 million was applied to the Lease CVR, reducing the respective liabilities.

The following table summarizes the components of the contingent value rights liability in the condensed consolidated balance sheet as of September 30, 2024 and December 31, 2023 (in thousands):

	September 30, 2024		December 31, 2023	
	Current	Non-Current	Current	Non-Current
Lease CVR	\$ 436	\$ 717	\$ 281	\$ 1,006
Intellectual Property CVR, net	—	(71)	—	—
Sales Tax CVR	332	—	—	—
Total CVR liability	\$ 768	\$ 646	\$ 281	\$ 1,006

As per the CVR Agreement, the total amount owed to CVR holders, after deductions permitted under the Merger Agreement, must be at least \$0.5 million to trigger a CVR payment prior to the end of the CVR term.

All other payments under the CVR Agreement were not considered probable and estimable as of September 30, 2024 and therefore no additional contingent consideration liability has been recorded. The Company will evaluate the probable and estimable range of outcomes under the CVR Agreement at each reporting period until the end of the CVR term and adjust the amounts accrued for as necessary.

#### ***Employment Agreements***

The Company has employment and consulting agreements with key personnel providing for compensation and severance in certain circumstances, as defined in the respective employment agreements.

#### ***Other Research and Development Arrangements***

As of September 30, 2024, the Company had standing agreements with consultants, contractors or service providers that generally can be terminated by the Company with 30 to 60 days written notice, unless otherwise indicated.

#### ***Litigation and Legal Proceedings***

The Company is subject to litigation and other claims that arise in the ordinary course of business. While the ultimate result of outstanding legal matters cannot presently be determined, the Company does not expect that the ultimate disposition will have a material effect on its results of financial condition, results of operations or cash flows. However, legal matters are inherently unpredictable and subject to significant uncertainties, some of which are beyond the Company's control. As such, there can be no assurance that the final outcome of any particular legal matter will not have a material adverse effect on the Company's financial condition results of operations or cash flows.

### **10. Licenses**

#### ***License Agreement with The University of North Carolina***

In May 2019, Neurogene entered into an Exclusive License Agreement with the University of North Carolina at Chapel Hill ("UNC") to obtain an exclusive, worldwide, royalty bearing license, with the right to grant sublicenses under certain patents to make, use, or sell products covered by such patents for prevention or treatment of disease or medical or genetic conditions, including CLN5 Batten disease or other diseases from dysfunction of the CLN5 gene. The Company is obligated to pay UNC up to \$1.7 million in sales-related milestones for licensed products based on annual sales of the licensed product in excess of defined thresholds and low single-digit percentage royalties on net sales of licensed product for as long as there is a valid patent claim under the patent rights. Neurogene is also required to reimburse any patent expenses, as well as pay a nonrefundable annual maintenance fee which, when royalties become due and payable, will be creditable against such royalties. During the year ended December 31, 2021, the FDA granted Orphan Drug Designation for CLN5 and the Company made a milestone payment of \$15,000 to UNC. During the year ended December 31, 2022, the Company dosed its first patient in a Phase 1 CLN5 study and made a milestone payment of \$30,000 to UNC. The annual license fee was \$4,000 for each of the nine months ended September 30, 2024 and 2023. In November 2024, the Company announced that it does not expect to move forward with the NGN-101 CLN5 Batten disease gene therapy program at this time due to the FDA's denial for the Company's RMAT application. The Company is currently evaluating options for the program.

#### ***License Agreement with The University of Edinburgh***

In December 2020, the University Court of the University of Edinburgh (the "University of Edinburgh") and Neurogene entered into the Master Collaboration Agreement ("MCA"). Under the MCA, Neurogene and the University of Edinburgh agreed to collaborate on certain research and development projects ("Projects") and Neurogene agreed to provide funding for such Projects for a 40-month initial term, which term was extended in November 2023 for an additional 33 months and may be further extended by mutual agreement. In exchange for such funding, the University of Edinburgh granted Neurogene the option to exclusively license any intellectual property arising from such Projects. If Neurogene exercises an exclusive option for a particular Project, Neurogene will enter into a separate exclusive license agreement on its own terms with the University of Edinburgh. Under the

MCA, Neurogene is obligated to pay semi-annual installment payments relating to funding of costs for personnel and lab consumables for the 40-month period. Either party may terminate the MCA for convenience upon 90 days' notice. If Neurogene terminates the MCA, it would be responsible for all non-cancellable costs and commitments related to any particular Project and any and all funding costs for any person working on such Project.

In March 2022, Neurogene exercised its option through the collaboration under the MCA and entered into a License Agreement (the "March 2022 Edinburgh License Agreement") with University of Edinburgh, pursuant to which Neurogene licensed certain patents and know-how related to the EXACT technology and optimized MECP2 cassettes on an exclusive basis. Under the March 2022 Edinburgh License Agreement, Neurogene obtained an exclusive, worldwide license to the licensed patents to develop, manufacture, supply, sell, and commercialize any products that utilize the licensed patents (the "Licensed Products") in exchange for low single-digit percentage royalties on future commercial net sales of the Licensed Products. Royalties are payable on a Licensed Product-by-Licensed Product and country-by-country basis until the latest of the expiration of the last licensed patent covering such Licensed Product in the country where the Licensed Product is sold, or, if no licensed patent exists or has expired in such country, then ten years from first commercial sale of such Licensed Product in such country. In connection with the license, Neurogene is also obligated to pay the University of Edinburgh up to \$5.3 million in regulatory-related milestones and up to \$25.0 million in sales-related milestones based on annual net sales of Licensed Products in excess of defined thresholds. During the nine months ended September 30, 2023, the Company expensed \$0.3 million for a milestone related to the first patient dosing in the Phase 1/2 trial for Rett syndrome.

For the nine months ended September 30, 2024 and 2023, the expense recorded by the Company related to the MCA was \$1.1 million and \$0.9 million, respectively.

***License Agreement with Virovek***

In September 2020, Neurogene entered into a Non-Exclusive License Agreement with Virovek, Inc., pursuant to which Neurogene has a license to use certain patents and know-how on a non-exclusive basis related to Neurogene's baculovirus ("baculo") process in exchange for low single-digit percentage royalties on future commercial net sales of each product using the baculo process, development milestone payments of up to \$0.2 million in the aggregate, and a nonrefundable annual license fee. During the nine months ended September 30, 2023, the Company recorded a milestone expense of \$0.1 million for the Rett Syndrome Investigational New Drug filing.

***License Agreement with Sigma-Aldrich Co***

In January 2023, Neurogene entered into a Non-Exclusive License Agreement with Sigma-Aldrich Co. LLC, pursuant to which Neurogene has a license to certain patents and know-how on a non-exclusive basis related to certain cell lines used in Neurogene's baculo process in exchange for a small annual fee on a product-by-product basis, payable once the first product candidate enters the clinic. In addition, on a product-by-product basis, Neurogene is obligated to pay up to \$2.5 million in the aggregate for development-related milestones. The Company recorded a license expense of \$60,000 for each of the nine months ended September 30, 2024 and 2023.

No expenses were recorded related to other in-process license agreements during the nine months ended September 30, 2024 and 2023. None will be due under these agreements unless and until certain development milestones are reached.

***License Agreement with Stanford***

In August 2024, the Company entered into a Nonexclusive License Agreement with the Board of Trustees of Leland Stanford Junior University (the "Stanford License Agreement") to license, on a non-exclusive basis, certain biological materials used by the Company in the manufacturing process of Neurogene's product candidates, including NGN-401. Over the 10-year term of the Stanford License Agreement, the Company is obligated to pay up to \$0.5 million in licensing fees. The Company recorded a license expense of \$50,000 for the nine months ended September 30, 2024.

## 11. Stockholders' Equity (Deficit)

### Common stock and pre-funded warrants

As of September 30, 2024, the Company is authorized to issue 450,000,000 shares of common stock with a par value of \$0.000001 per share.

The Company has pre-funded warrants outstanding to purchase an aggregate of 3,959,954 shares of common stock as of September 30, 2024. The pre-funded warrants are exercisable at any time for an exercise price of \$0.000001, except that the pre-funded warrants cannot be exercised by a holder if, after giving effect thereto, such holder would beneficially own more than 9.99% of the outstanding common stock, subject to certain exceptions. However, any holder may increase or decrease such percentage to any other percentage (not in excess of 19.99%) upon at least 61 days' prior notice from the holder to the Company. The holders of the pre-funded warrants will not have the right to vote the shares underlying the pre-funded warrants on any matter except to the extent required by Delaware law. These warrants were classified as equity.

During the nine months ended September 30, 2024, 103,407 shares of common stock were issued upon the exercise of pre-funded warrants. Proceeds of the exercise to the Company were immaterial.

The Company had reserved shares of common stock for future issuance as follows:

	September 30, 2024	December 31, 2023
Unvested restricted stock units	477,613	—
Options outstanding	1,384,182	823,833
Shares available for future grant under the 2023 Equity Incentive Plan	1,468,403	2,237,722
Total common stock reserved	<u>3,330,198</u>	<u>3,061,555</u>

## 12. Stock-Based Compensation

The Company measures stock-based awards at their grant-date fair value and records compensation expense on a straight-line basis over the vesting period of the awards. The Company recorded stock-based compensation expense in the following expense categories in its accompanying statements of operations (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Research and development	\$ 1,272	\$ 242	\$ 3,212	\$ 657
General and administrative	1,202	144	2,621	406
Total expense	<u>\$ 2,474</u>	<u>\$ 386</u>	<u>\$ 5,833</u>	<u>\$ 1,063</u>

The following table summarizes the option activity:

	Number of shares	Weighted average exercise price per share	Weighted average remaining contractual term (years)
Outstanding at December 31, 2023	823,833	\$ 31.43	5.89
Granted	849,012	\$ 35.31	
Exercised *	(83,795)	\$ 17.63	
Expired/Forfeited	(204,868)	\$ 74.86	
Outstanding at September 30, 2024	<u>1,384,182</u>	\$ 28.22	8.09
Exercisable at September 30, 2024	<u>461,339</u>	\$ 18.34	6.02

\* 14,582 options were exercised on September 30, 2024 and the cash for the option exercise was received on October 1, 2024. Accordingly, a receivable of approximately \$0.3 million was recorded as a reduction in additional paid-in-capital as of September 30, 2024 in the condensed consolidated balance sheet.

As of September 30, 2024, the aggregate intrinsic value of outstanding options and exercisable options was approximately \$20.0 million and \$11.8 million, respectively. The aggregate intrinsic value of options exercised was approximately \$1.4 million for the nine months ended September 30, 2024.

The weighted-average grant date fair value of options granted was \$26.52 and \$12.99 per share for the nine months ended September 30, 2024 and 2023, respectively. The Company recorded stock-based compensation related to stock options of approximately \$1.8 million and \$0.4 million for the three months ended September 30, 2024 and 2023, respectively, and \$4.3 million and \$1.0 million for the nine months ended September 30, 2024 and 2023, respectively. As of September 30, 2024, the total unrecognized compensation expense related to unvested stock option awards was approximately \$19.9 million, which the Company expects to recognize over a weighted-average period of 3.08 years.

The fair value of each option was estimated on the grant date using the weighted average assumptions in the table below:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Expected volatility	90.85%-92.31%	—%—%	86.39%-92.31%	82.96%-83.70%
Risk-free interest rate	3.43%-4.40%	—%—%	3.43%-4.60%	3.45% - 4.46%
Expected life (in years)	5.95-6.12	0.00-0.00	5.50-6.15	3.58 - 6.08
Expected dividend yield	—	—	—	—
Fair value of common stock <sup>(1)</sup>				\$ 18.39

(1) Prior to the reverse merger, the Company periodically estimated the fair value of the Company's common stock considering, among other things, valuations of its common stock prepared by management with the assistance of a third-party valuation firm in accordance with the guidance provided by the American Institute of Certified Public Accountants 2013 Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. Following the reverse merger, the fair value of the Company's common stock is based on the closing stock price on the date of grant as reported on the Nasdaq Global Market.

A summary of the Company's RSU activity and related information for the nine months ended September 30, 2024 is as follows:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested at December 31, 2023	—	\$ —
Restricted stock units granted	482,384	\$ 36.06
Restricted stock units forfeited	(4,771)	\$ 36.06
Unvested at September 30, 2024	477,613	\$ 36.06

252,124 of these RSUs were granted with vesting in two equal tranches based on certain performance conditions ("PSUs"). Each PSU entitles the holder to receive one share of the Company's common stock when the PSU vests. During the nine months ended September 30, 2024, the related performance conditions were not met and are not currently considered probable to vest. As such, no expense is recognized as of September 30, 2024.

The Company recorded stock-based compensation expense related to RSUs of approximately \$0.7 million and \$1.5 million for the three and nine months ended September 30, 2024, respectively. As of September 30, 2024, there was approximately \$6.6 million of unrecognized compensation cost related to unvested RSUs, which is expected to be recognized over a remaining weighted average vesting period of approximately 2.44 years.

### **13. Subsequent Events**

From October 1, 2024 until the financial statements were issued, the Company granted 34,695 options to employees and 8,858 options were exercised.

#### ***2024 private placement***

On November 5, 2024, the Company closed a private investment in public equity financing in which the Company sold 1,835,000 shares of common stock at a purchase price of \$50.00 per share and, in lieu of shares of common stock, pre-funded warrants to purchase up to an aggregate of 2,165,042 shares of common stock, at a purchase price of \$49.999 per pre-funded warrant, to certain institutional accredited investors. The pre-funded warrants are immediately exercisable until exercised in full at a price of \$0.001 per share of common stock. The aggregate gross proceeds to the Company are approximately \$200.0 million. Net proceeds, after deducting commissions and other offering expenses, are expected to total approximately \$189.5 million.



**PART II**  
**INFORMATION NOT REQUIRED IN PROSPECTUS**

**Item 13. Other Expenses of Issuance and Distribution**

The expenses payable by us in connection with the issuance and distribution of the securities being registered hereunder on Form S-1 (other than underwriting discounts and commissions, if any) are set forth below. The Selling Stockholders will not bear any portion of such expenses. Each item listed is estimated, except for the SEC registration fee:

SEC registration fee	\$ \$	13,197
Printing and engraving		90,000
Legal fees and expenses		250,000
Accounting fees and expenses		75,000
Miscellaneous expenses		10,000
Total	\$ \$	<u>438,197</u>

**Item 14. Indemnification of Officers and Directors**

Section 145 of the DGCL authorizes a court to award, or a corporation's board of directors to grant, indemnity to directors and officers under certain circumstances and subject to certain limitations. The terms of Section 145 of the DGCL are sufficiently broad to permit indemnification under certain circumstances for liabilities, including reimbursement of expenses incurred, arising under the Securities Act.

As permitted by the DGCL, the Registrant's Certificate of Incorporation contains provisions that eliminate the personal liability of its directors for monetary damages for any breach of fiduciary duties as a director to the fullest extent permitted under applicable law.

As permitted by the DGCL, the Registrant's Bylaws provide that:

- the Registrant is required to indemnify its directors and executive officers to the fullest extent permitted by the DGCL, subject to very limited exceptions;
- the Registrant may indemnify its other employees and agents as set forth in the DGCL;
- the Registrant is required to advance expenses, as incurred, to its directors and executive officers in connection with a legal proceeding to the fullest extent permitted by the DGCL, subject to very limited exceptions; and
- the rights conferred in the Bylaws are not exclusive.

The Registrant has entered, and intends to continue to enter, into separate indemnification agreements with its directors and executive officers to provide these directors and executive officers additional contractual assurances regarding the scope of the indemnification set forth in the Registrant's Certificate of Incorporation and Bylaws and to provide additional procedural protections. At present, there is no pending litigation or proceeding involving a director or executive officer of the Registrant regarding which indemnification is sought.

The indemnification provisions in the Registrant's Certificate of Incorporation, Bylaws and the indemnification agreements entered into or to be entered into between the Registrant and each of its directors and executive officers may be sufficiently broad to permit indemnification of the Registrant's directors and executive officers for liabilities arising under the Securities Act.

The Registrant currently carries liability insurance for its directors and officers.

**Item 15. Recent Sales of Unregistered Securities**

On November 5, 2024, we completed a private placement of shares of Common Stock and the 2024 Pre-Funded Warrants to support the completion of enrollment of a future registrational study for NGN-401 for Rett syndrome, CMC scale-up to support NGN-401 registrational activities, further development of our EXACT™ gene therapy pipeline and other general corporate purposes. These securities were sold pursuant to the Securities Purchase Agreement. Pursuant to and subject to the terms and conditions of a Securities Purchase Agreement between Neurogene and the Selling Stockholders named there in, Neurogene issued and sold, and Selling Stockholders named in the Securities Purchase Agreement purchased 1,835,000 shares of Common Stock and 2,165,042 2024 Pre-Funded Warrants, exercisable for 2,156,042 shares of Common Stock, at a purchase price of \$50.00 per share for the Common Stock or \$49.999 per 2024 Pre-Funded Warrant, which represents the per share purchase price of the Common Stock less the \$0.001 per share exercise price for each 2024 Pre-Funded Warrant, for an aggregate purchase price of approximately \$200.0 million. The issuance was made in a transaction not involving a public offering pursuant to an exemption from the registration requirements of the Securities Act, in reliance upon Section 4(a)(2) of the Securities Act and/or Rule 506 of Regulation D promulgated thereunder, or under any state securities laws.

## Item 16. Exhibits and Financial Statement Schedules

### (a) Exhibits

<u>Exhibit</u>	<u>Description</u>
2.1†	<a href="#">Agreement and Plan of Merger, dated as of July 17, 2023, by and among Neoleukin Therapeutics, Inc., Project North Merger Sub, Inc. and Neurogene Inc. (Incorporated by reference to Exhibit 2.1 to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on July 18, 2023).</a>
3.1	<a href="#">Amended and Restated Certificate of Incorporation of the Company, filed December 18, 2023 (Incorporated by reference to Exhibit 3.2 of the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on December 19, 2023).</a>
3.2	<a href="#">Amended and Restated Bylaws of the Company (Incorporated by reference to Exhibit 3.3 of the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on December 19, 2023).</a>
3.3	<a href="#">Certificate of Designation of Preferences, Rights and Limitations of Series A Convertible Stock of Neurogene Inc. filed August 8, 2019 (Incorporated by reference to Exhibit 3.1 of the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission August 12, 2019).</a>
4.1	<a href="#">Specimen Common Stock Certificate of Neurogene Inc. (Incorporated by reference to Exhibit 4.1 to the Registrant's Quarterly Report on Form 10-Q for the quarter ended March 31, 2014 filed with the Securities and Exchange Commission on May 13, 2014).</a>
4.2	<a href="#">Registration Rights Agreement, dated September 19, 2016, by and between Aquinox Pharmaceuticals, Inc. and the persons listed on Schedule A attached thereto (Incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on September 20, 2016).</a>
4.3	<a href="#">Form of Registration Rights Agreement (Incorporated by reference to Exhibit 10.2 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on November 4, 2024).</a>
4.4	<a href="#">Form of Pre-Funded Warrant (Incorporated by reference to Exhibit 4.1 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on December 19, 2019).</a>
4.5	<a href="#">Form of Pre-Funded Warrant (Incorporated by reference to Exhibit 4.1 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on July 2, 2020).</a>
4.6	<a href="#">Form of Pre-Funded Warrant (Incorporated by reference to Exhibit A of Exhibit C to Exhibit 2.1 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on July 18, 2023).</a>
4.7	<a href="#">Form of Pre-Funded Warrant (Incorporated by reference to Exhibit 4.1 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on November 4, 2024).</a>
5.1	<a href="#">Opinion of Gibson, Dunn &amp; Crutcher LLP.</a>

<b>Exhibit</b>	<b>Description</b>
10.1	<a href="#">Lease Agreement, dated September 23, 2019, by and between Neoleukin Therapeutics, Inc. and ARE-Eastlake Avenue No. 3, LLC (Incorporated by reference to Exhibit 10.7 to Registrant’s Quarterly Report on Form 10-Q for the quarter ended September 30, 2019 filed with the Securities and Exchange Commission on November 13, 2019).</a>
10.2	<a href="#">Lease Agreement, dated December 23, 2019, by and between Neoleukin Therapeutics, Inc. and ARE Eastlake Avenue No. 3, LLC (Incorporated by reference to Exhibit 10.8 to Registrant’s Annual Report on Form 10-K for the year ended December 31, 2019 filed with the Securities and Exchange Commission on March 12, 2020).</a>
10.3	<a href="#">First Amendment to Lease, dated June 18, 2020, by and between Neoleukin Therapeutics, Inc. and ARE-Eastlake Avenue No. 3, LLC (Incorporated by reference to Exhibit 10.1 to Registrant’s Quarterly Report on Form 10-Q for the quarter ended June 30, 2020 filed with the Securities and Exchange Commission on August 12, 2020).</a>
10.4	<a href="#">First Amendment to Lease, dated November 5, 2020, by and between Neoleukin Therapeutics, Inc. and ARE-Seattle No. 28, LLC (Incorporated by reference to Exhibit 10.24 to Registrant’s Annual Report on Form 10-K for the year ended December 31, 2020 filed with the Securities and Exchange Commission on March 25, 2021).</a>
10.5	<a href="#">Second Amendment to Lease, dated March 24, 2021, by and between Neoleukin Therapeutics, Inc. and ARE-Seattle No. 28, LLC (Incorporated by reference to Exhibit 10.1 to Registrant’s Quarterly Report on Form 10-Q for the quarter ended March 31, 2021 filed with the Securities and Exchange Commission on May 12, 2021).</a>
10.6	<a href="#">Contingent Value Rights Agreement, dated December 18, 2023, by and among Neoleukin Therapeutics, Inc., Equiniti Trust Company, LLC and Donna Cochener (Incorporated by reference to Exhibit 10.6 of Registrant’s Current Report on Form 8-K filed with the Securities and Exchange Commission on December 19, 2023).</a>
10.8	<a href="#">Letter Agreement, dated July 17, 2023, by and between Neoleukin Therapeutics, Inc. and Baker Bros. Advisors LP (Incorporated by reference to Exhibit 10.5 to Registrant’s Current Report on Form 8-K filed with the Securities and Exchange Commission on July 18, 2023).</a>
10.9††	<a href="#">Master Research Collaboration Agreement, dated December 4, 2020, by and between Neurogene Inc. and The University Court of The University of Edinburgh (Incorporated by reference to Exhibit 10.29 to Registrant’s Registration Statement on Form S-4/A filed with the Securities and Exchange Commission on September 28, 2023).</a>
10.10	<a href="#">Amendment 1 to the Master Research Collaboration Agreement, dated November 29, 2023, by and between Neurogene Inc. and The University Court of The University of Edinburgh (Incorporated by reference to Exhibit 10.19 to Registrant’s Current Report on Form 8-K filed with the Securities and Exchange Commission on December 19, 2023).</a>
10.11††	<a href="#">Option Agreement, dated January 7, 2020, by and between Neurogene Inc. and the University Court of the University of Edinburgh (Incorporated by reference to Exhibit 10.30 to Registrant’s Registration Statement on Form S-4/A filed with the Securities and Exchange Commission on September 28, 2023).</a>
10.12††	<a href="#">Exclusive License Agreement, dated May 16, 2019, by and between The University of North Carolina at Chapel Hill and Neurogene Inc. (Incorporated by reference to Exhibit 10.32 to Registrant’s Registration Statement on Form S-4/A filed with the Securities and Exchange Commission on September 28, 2023).</a>
10.13††	<a href="#">License Agreement, dated March 4, 2022, by and between The University Court of the University of Edinburgh and Neurogene Inc. (Incorporated by reference to Exhibit 10.31 to the Registrant’s Registration Statement on Form S-4/A filed with the Securities and Exchange Commission on September 28, 2023).</a>
10.14††	<a href="#">Non-Exclusive License Agreement, dated September 30, 2020, by and between Neurogene Inc. and Virovek, Inc. (Incorporated by reference to Exhibit 10.33 to the Registrant’s Registration Statement on Form S-4/A filed with the Securities and Exchange Commission on September 28, 2023).</a>
10.15††	<a href="#">Non-Exclusive License Agreement, dated January 19, 2023, by and between Sigma-Aldrich Co. LLC and Neurogene Inc. (Incorporated by reference to Exhibit 10.34 to the Registrant’s Registration Statement on Form S-4/A filed with the Securities and Exchange Commission on September 28, 2023).</a>

<b>Exhibit</b>	<b>Description</b>
10.16	<a href="#">Lease Agreement, dated August 4, 2019, by and between Stella Link Investments, Ltd. and Neurogene Inc as amended by that certain First Amendment to Lease Agreement, dated September 17, 2020, by and between Stella Link Investments, Ltd. and Neurogene Inc. (Incorporated by reference to Exhibit 10.35 to Registrant's Registration Statement on Form S-4/A filed with the Securities and Exchange Commission on September 28, 2023).</a>
10.17	<a href="#">Sublease Agreement, dated May 16, 2019, by and between GPB Capital Holdings, LLC and Neurogene Inc., as amended and assumed pursuant to that certain Assumption and Attornment of Lease and Release Agreement, dated July 30, 2021, by and among GTM Associates, LLC, GPB Capital Holdings, LLC and Neurogene Inc., as further amended by that certain Amendment to Attorned Sublease, dated February 22, 2022, by and between GTM Associates, LLC and Neurogene Inc. (Incorporated by reference to Exhibit 10.36 to Registrant's Registration Statement on Form S-4/A filed with the Securities and Exchange Commission on September 28, 2023).</a>
10.18	<a href="#">Form of Indemnification Agreement entered into between Neurogene Inc. and each of its directors and its executive officers (Incorporated by reference to Exhibit 10.20 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on December 19, 2023).</a>
10.19#	<a href="#">Neurogene Inc. 2018 Equity Incentive Plan (Incorporated by reference to Exhibit 99.1 to Registrant's Registration Statement on Form S-8 filed with the Securities and Exchange Commission on February 20, 2024).</a>
10.20#	<a href="#">Neurogene Inc. 2023 Equity Incentive Plan (Incorporated by reference to Exhibit 99.2 to Registrant's Registration Statement on Form S-8 filed with the Securities and Exchange Commission on February 20, 2024).</a>
10.21#	<a href="#">Neurogene Inc. 2023 Employee Stock Purchase Plan (Incorporated by reference to Exhibit 99.3 to Registrant's Registration Statement on Form S-8 filed with the Securities and Exchange Commission on February 20, 2024).</a>
10.22#	<a href="#">Neoleukin Therapeutics, Inc. Amended and Restated 2014 Equity Incentive Plan as amended and restated on May 13, 2021 (Incorporated by reference to Exhibit 10.1 to Registrant's 8-K filed with the Securities and Exchange Commission on May 14, 2021).</a>
10.23#	<a href="#">Form of Separation Agreement, by and between Neoleukin Therapeutics, Inc. and Donna Cochener (Incorporated by reference to Exhibit 10.23 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on December 19, 2023).</a>
10.24#	<a href="#">Form of Separation Agreement, by and between Neoleukin Therapeutics, Inc. and Sean Smith (Incorporated by reference to Exhibit 10.24 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on December 19, 2023).</a>
10.26	<a href="#">Form of Subscription Agreement (Incorporated by reference to Exhibit A to Exhibit 2.1 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on July 18, 2023).</a>
10.27	<a href="#">Securities Purchase Agreement dated November 4, 2024 by and among the Company and each purchaser listed on the signature page thereto (Incorporated by reference to Exhibit 10.1 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on November 4, 2024).</a>
10.29	<a href="#">Executive Employment Agreement with Rachel McMinn dated April 1, 2024 (Incorporated by reference to Exhibit 10.1 to Registrant's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 10, 2024).</a>
10.30	<a href="#">Executive Employment Agreement with Christine Mikail Cvijic dated April 1, 2024 (Incorporated by reference to Exhibit 10.2 to Registrant's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 10, 2024).</a>
10.31	<a href="#">Amended and Restated Consulting Agreement with Stuart Cobb Consulting Ltd. dated April 19, 2024 (Incorporated by reference to Exhibit 10.3 to Registrant's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 10, 2024).</a>
10.32††	<a href="#">Nonexclusive License Agreement between Neurogene and the Board of Trustees of the Leland Stanford Junior University dated August 6, 2024 (Incorporated by reference to Exhibit 10.1 to Registrant's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 18, 2024).</a>
21.1	<a href="#">List of Subsidiaries of Neurogene Inc. (Incorporated by reference to Exhibit 21.1 to Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on December 19, 2023).</a>
23.1	<a href="#">Consent of Ernst &amp; Young LLP</a>

<u>Exhibit</u>	<u>Description</u>
23.2	<a href="#">Consent of Deloitte &amp; Touche LLP</a>
24.1	<a href="#">Power of Attorney (included on the signature page to the registration statement)</a>
101.INS	Inline XBRL Instance Document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document.
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	Inline XBRL Taxonomy Extension Labels Linkbase Document.
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).
107	<a href="#">Filing Fee Table</a> .

† Indicates a management contract or compensatory plan.

†† Portions of this exhibit (indicated by asterisk) have been omitted in accordance with the rules of the Securities and Exchange Commission.

# Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

**(b) Financial statement schedules**

None.

**Item 17. Undertakings**

(a) The undersigned Registrant hereby undertakes:

- (1) To file, during any period in which offers or sales are being made, a post-effective amendment to this registration statement:
  - (i) to include any prospectus required by Section 10(a)(3) of the Securities Act;
  - (ii) to reflect in the prospectus any facts or events arising after the effective date of the registration statement (or the most recent post-effective amendment thereof) which, individually or in the aggregate, represent a fundamental change in the information set forth in the registration statement. Notwithstanding the foregoing, any increase or decrease in volume of securities offered (if the total dollar value of securities offered would not exceed that which was registered) and any deviation from the low or high end of the estimated maximum offering range may be reflected in the form of prospectus filed with the SEC pursuant to Rule 424(b) if, in the aggregate, the changes in volume and price represent no more than 20 percent change in the maximum aggregate offering price set forth in the “Filing Fee Table” in the effective registration statement; and
  - (iii) to include any material information with respect to the plan of distribution not previously disclosed in the registration statement or any material change to such information in the registration statement;
- (2) That, for the purpose of determining any liability under the Securities Act, each such post-effective amendment shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial *bona fide* offering thereof.
- (3) To remove from registration by means of a post-effective amendment any of the securities being registered which remain unsold at the termination of the offering.
- (4) That, for the purpose of determining liability under the Securities Act to any purchaser, each prospectus filed pursuant to Rule 424(b) as part of a registration statement relating to an offering, other than registration statements relying on Rule 430B or other than prospectuses filed in reliance on Rule

430A, shall be deemed to be part of and included in the registration statement as of the date it is first used after effectiveness. *Provided, however,* that no statement made in a registration statement or prospectus that is part of the registration statement or made in a document incorporated or deemed incorporated by reference into the registration statement or prospectus that is part of the registration statement will, as to a purchaser with a time of contract of sale prior to such first use, supersede or modify any statement that was made in the registration statement or prospectus that was part of the registration statement or made in any such document immediately prior to such date of first use.

- (5) Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers, and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer, or controlling person of the Registrant in the successful defense of any action, suit, or proceeding) is asserted by such director, officer, or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

## SIGNATURES

Pursuant to the requirements of the Securities Act, the Registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of New York, State of New York, on December 2, 2024.

### NEUROGENE INC.

By:                   /s/ Rachel McMinn  
Rachel McMinn, Ph.D.  
Chief Executive Officer

## POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below hereby constitutes and appoints Rachel McMinn and Christine Mikail, as his or her true and lawful attorneys-in-fact, proxies and agents, each with full power of substitution, for him or her in any and all capacities, to sign any and all amendments to this registration statement (including post-effective amendments or any abbreviated registration statement and any amendments thereto filed pursuant to Rule 462(b) increasing the number of securities for which registration is sought), and to file the same, with all exhibits thereto and other documents in connection therewith, with the SEC, granting unto said attorneys-in-fact, proxies and agents full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully for all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact, proxies and agents, or their or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

### Signatures

Pursuant to the requirements of the Securities Act, the Registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the city of New York, State of New York, on the date indicated.

Date: December 2, 2024

Neurogene Inc.  
By:                   /s/ Rachel McMinn  
Name:                   Rachel McMinn, Ph.D.  
Title:                   Chief Executive Officer

Pursuant to the requirements of the Securities Act, this registration statement has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
<u>/s/ Rachel McMinn</u> Rachel McMinn, Ph.D.	Chief Executive Officer, Director	December 2, 2024
<u>/s/ Christine Mikail</u> Christine Mikail, J.D.	President and Chief Financial Officer	December 2, 2024
<u>/s/ Robert Baffi</u> Robert Baffi, Ph.D.	Director	December 2, 2024
<u>/s/ Cory Freedland</u> Cory Freedland, Ph.D.	Director	December 2, 2024
<u>/s/ Sarah B. Noonberg</u> Sarah B. Noonberg, M.D., Ph.D.	Director	December 2, 2024
<u>/s/ Rohan Palekar</u> Rohan Palekar	Director	December 2, 2024
<u>/s/ Robert Keith Woods</u> Robert Keith Woods	Director	December 2, 2024



**Calculation of Filing Fee Tables**  
**FORM S-1**  
(Form Type)

**Neurogene Inc.**  
(Exact Name of Registrant as Specified in its Charter)

**Table 1: Newly Registered and Carry Forward Securities**

	Security Type	Security Class Title	Fee Calculation or Carry Forward Rule	Amount Registered	Proposed Maximum Offering Price Per Unit	Maximum Aggregate Offering Price	Fee Rate	Amount of Registration Fee	Carry Forward Form Type	Carry Forward File Number	Carry Forward Initial effective date	Filing Fee Previously Paid In Connection with Unsold Securities to be Carried Forward
<b>Newly Registered Securities</b>												
Fees to Be Paid	Equity	Common stock, par value \$0.000001 per share(1)	Rule 457(c)	4,000,042 (2)	\$86,198,105.07 (3)	\$21.5493 (3)	0.00015310	\$13,196.93	—	—	—	—
Fees Previously Paid	—	—	—	—	—	—	—	—	—	—	—	—
<b>Carry Forward Securities</b>												
Carry Forward Securities	N/A	N/A	N/A	N/A		N/A			N/A	N/A	N/A	N/A
<b>Total Offering Amounts</b>						\$86,198,105.07		\$13,196.93				
<b>Total Fees Previously Paid</b>								—				
<b>Total Fee Offsets</b>								—				
<b>Net Fee Due</b>								\$13,196.93				

- (1) Pursuant to Rule 416 under the Securities Act of 1933, as amended, this registration statement also covers such an indeterminate amount of shares of common stock as may become issuable to prevent dilution resulting from stock splits, stock dividends and similar events.
- (2) The amount registered consists of up to (i) 1,835,000 shares of Common Stock held by certain selling stockholders and (ii) 2,165,042 shares of Common Stock issuable upon the exercise of pre-funded warrants held by certain selling stockholders.
- (3) Estimated solely for the purpose of calculating the registration fee, based on the average of the high and low prices of the shares of common stock on The Nasdaq Global Market on November 25, 2024 (such date being within five business days of the date that this registration statement was first filed with the Securities and Exchange Commission, in accordance with Rule 457(c) under the Securities Act).

November [●], 2024

Neurogene Inc.  
535 W 24<sup>th</sup> St.  
5<sup>th</sup> Floor  
New York, NY 10011

Re: Neurogene Inc.  
Registration Statement on Form S-1

Ladies and Gentlemen:

We have acted as counsel to Neurogene Inc., a Delaware corporation (the “Company”), in connection with the preparation and filing with the Securities and Exchange Commission (the “Commission”) of a Registration Statement on Form S-1 (the “Registration Statement”) pursuant to the Securities Act of 1933, as amended (the “Securities Act”), relating to the resale from time to time by the selling stockholders named therein of (i) up to 1,835,000 shares (the “Shares”) of the Company’s common stock, par value \$0.000001 per share (“Common Stock”), and (ii) 2,165,042 shares of Common Stock (the “Pre-Funded Warrant Shares”) issuable upon the exercise of pre-funded warrants (the “Pre-Funded Warrants”).

In arriving at the opinion expressed below, we have examined originals, or copies certified or otherwise identified to our satisfaction as being true and complete copies of the originals, of the specimen common stock certificates, form of Pre-Funded Warrant and such other documents, corporate records, certificates of officers of the Company and of public officials and other instruments as we have deemed necessary or advisable to enable us to render these opinions. In our examination, we have assumed without independent investigation the genuineness of all signatures, the legal capacity and competency of all natural persons, the authenticity of all documents submitted to us as originals and the conformity to original documents of all documents submitted to us as copies.

Based on the foregoing, and subject to the assumptions, exceptions, qualifications and limitations set forth herein, we are of the opinion that (i) the Shares are validly issued, fully paid and non-assessable, (ii) provided that the Pre-Funded Warrants have been duly executed and delivered by the Company and duly delivered to the purchaser thereof against payment therefor, then the Pre-Funded Warrants, when issued and sold as described in the Registration Statement, assuming a sufficient number of Pre-Funded Warrant Shares are at the time available for issuance, will be a valid and legally binding obligation of the Company, enforceable against the Company in accordance with its terms, except as enforcement thereof may be limited by bankruptcy, insolvency, reorganization, moratorium or other similar laws relating to or affecting creditors’ rights generally and by general equitable principles (regardless of whether such enforceability is considered in a proceeding at law or in equity) and implied covenants of good faith and fair dealing, and (iii) the Pre-Funded Warrant Shares, when issued upon a valid exercise of the Pre-Funded Warrants in accordance with their terms, will have been duly authorized and validly issued and will be fully paid and non-assessable.

We consent to the filing of this opinion as an exhibit to the Registration Statement, and we further consent to the use of our name under the caption “Legal Matters” in the Registration Statement and the prospectus that forms a part thereof. In giving these consents, we do not thereby admit that we are within the category of persons whose consent is required under Section 7 of the Securities Act or the rules and regulations of the Commission.

Very truly yours,

/s/ Gibson, Dunn & Crutcher LLP

**Consent of Independent Registered Public Accounting Firm**

We consent to the reference to our firm under the caption "Experts" and to the use of our report dated August 18, 2023 (except for the effects of the exchange ratio disclosed in Note 1, sub-section Reverse Merger and Pre-Closing Financing, as to which the date is March 18, 2024), in the Registration Statement (Form S-1 No. 333-XXXXXX) and related Prospectus of Neurogene Inc. for the registration of 4,000,042 shares of its common stock.

/s/ Ernst & Young LLP

Stamford, Connecticut

December 2, 2024

**CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

We consent to the use in this Registration Statement on Form S-1 of our report dated March 18, 2024, relating to the financial statements of Neurogene Inc. We also consent to the reference to us under the heading "Experts" in such Registration Statement.

Seattle, Washington

December 2, 2024