



Neurogene Reports Fourth Quarter and Full Year 2023 Financial Results and Highlights Recent Updates

March 18, 2024

Expanded Phase 1/2 gene therapy trial for Rett syndrome to inform future registrational study design; Company remains on track to share interim clinical data in 4Q:24

Strong financial position with runway into 2H:26 following reverse merger and private financing in December 2023

NEW YORK--(BUSINESS WIRE)--Mar. 18, 2024-- Neurogene Inc. (Nasdaq: NGNE), a clinical-stage company founded to bring life-changing genetic medicines to patients and families affected by rare neurological diseases, today announced fourth quarter and full year 2023 financial results and highlighted recent corporate updates.

"We started the year with strong execution in our Phase 1/2 NGN-401 gene therapy trial for female pediatric patients with Rett syndrome, meeting our goal to expand the trial and add a high-dose cohort. We believe this expanded dataset will support future regulatory discussions to align on the design of a potential registrational study," said Rachel McMinn, Ph.D., Chief Executive Officer and Founder of Neurogene. "NGN-401 has the potential to be a best-in-class treatment option with a targeted route of administration to deliver full-length, functional copies of the *MECP2* gene. We are pleased that NGN-401 has been well-tolerated in all three patients dosed, with no signs of overexpression-related toxicity, demonstrating the potential of our EXACT transgene regulation technology to constrain *MECP2* levels within a narrow and therapeutically relevant range. As we look ahead, we remain on track for interim clinical data from the NGN-401 trial in the fourth quarter of this year."

Continued Dr. McMinn, "Our decision to execute a reverse merger, along with the concurrent private financing, provides us with cash runway into the second half of 2026, beyond key inflection points in our NGN-401 program and our NGN-101 program for CLN5 Batten disease. We look forward to sharing data from both programs later this year and continuing to advance our pipeline to enable an additional development candidate using EXACT in 2025."

Fourth Quarter 2023 and Recent Highlights, and Anticipated Milestones

Phase 1/2 Trial of NGN-401 Gene Therapy for Treatment of Rett Syndrome

- As previously planned, amended the protocol, [expanding the trial](#) to include more patients in low-dose Cohort 1 and added a high-dose Cohort 2; these updates are expected to provide a robust dataset and inform the design of a future registrational study
- Remains on track to report interim clinical data from Cohort 1 in the fourth quarter of 2024; additional interim data, including from Cohort 2, are expected in the second half of 2025
- Dosed the third patient early in the first quarter of 2024; removed the dosing stagger in Cohort 1, enabling the remaining patients in the cohort to be dosed in parallel
- Plans to complete enrollment in Cohort 1 in the second half of 2024; enrollment in Cohort 2 is expected to begin in the second quarter of 2024
- Reported that NGN-401 has been generally well-tolerated and there have been no treatment-emergent or procedure-related serious adverse events or signs of overexpression-related toxicity observed in any patient
- Received NGN-401 clinical trial application approval from the United Kingdom ("UK") Medicines and Healthcare products Regulatory Agency and working to on-board clinical sites in the UK

Phase 1/2 Trial of NGN-101 Gene Therapy for Treatment of CLN5 Batten Disease

- Continuing enrollment in high-dose Cohort 3, and expects to provide interim clinical data and regulatory update in the second half of 2024

Additional Corporate Updates

- Expanded the leadership team with appointment of [Julie Jordan, M.D.](#), as Chief Medical Officer; Dr. Jordan brings more than 20 years of experience to Neurogene, including design and execution of clinical trials for central nervous system disorders and gene therapies
- Completed reverse merger and concurrent private placement of \$95 million, and began trading on the Nasdaq Global Market under "NGNE" in mid-December 2023
- Continues to expect to advance one additional product candidate, using EXACT technology, from the discovery stage into

the clinic in 2025

Fourth Quarter and Full Year 2023 Financial Results

- **Cash and Cash Equivalents:** Cash, cash equivalents and investments as of December 31, 2023 were \$197.2 million.
- **Research & Development (“R&D”) Expenses:** R&D expenses were \$12.2 million and \$44.4 million for the three and twelve months ended December 31, 2023, respectively, compared to \$11.0 million and \$47.5 million for the three and twelve months ended December 31, 2022, respectively. The decrease in R&D expenses for the twelve months ended December 31, 2023 was primarily driven by a decrease in manufacturing and development and pre-clinical costs offset by increased clinical trial costs.
- **General & Administrative (“G&A”) Expenses:** G&A expenses were \$2.5 million and \$11.2 million for the three and twelve months ended December 31, 2023, respectively, compared to \$2.0 million and \$9.0 million for the three and twelve months ended December 31, 2022, respectively. The increase in G&A expenses for the twelve months ended December 31, 2023 was primarily driven by an increase in personnel costs, increased professional and consulting fees related to the reverse merger, and increased insurance and information technology costs. Due to the timing of the closing of the reverse merger in mid-December 2023, additional transaction-related expenses are expected to be recognized in the first quarter of 2024.
- **Net Income and Net Loss:** Net income was \$2.4 million and net loss was \$36.3 million for the three and twelve months ended December 31, 2023, respectively, compared to net loss of \$12.3 million and \$55.2 million for the three and twelve months ended December 31, 2022, respectively. Net income for the three months ended December 31, 2023 included a one-time \$16.4 million bargain purchase gain related to the reverse merger.

Upcoming Events

- Stifel 2024 Virtual CNS Days: Management will participate in a fireside chat on Wednesday, March 20 at 9:30 a.m. ET

About Neurogene

The mission of Neurogene is to treat devastating neurological diseases to improve the lives of patients and families impacted by these rare diseases. Neurogene is developing novel approaches and treatments to address the limitations of conventional gene therapy in central nervous system disorders. This includes selecting a delivery approach to maximize distribution to target tissues and designing products to maximize potency and purity for an optimized efficacy and safety profile. The Company’s novel and proprietary EXACT transgene regulation platform technology allows for the delivery of therapeutic levels while limiting transgene toxicity associated with conventional gene therapy. Neurogene has constructed a state-of-the-art gene therapy manufacturing facility in Houston, Texas. CGMP production of NGN-401 was conducted in this facility and will support pivotal clinical development activities. For more information, visit www.neurogene.com.

Cautionary Note Regarding Forward-Looking Statements

Statements in this press release which are not historical in nature are intended to be, and hereby are identified as, forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. These statements may discuss goals, intentions and expectations as to future plans, trends, events, results of operations or financial condition, or otherwise, based on current expectations and beliefs of the management of Neurogene, as well as assumptions made by, and information currently available to, management of Neurogene, including, but not limited to, statements regarding: the therapeutic potential and utility, efficacy and clinical benefits of NGN-401; the safety and tolerability profile of NGN-401; trial designs, clinical development plans and timing for NGN-401, including anticipated timing of enrollment in and clinical trial results from the Company’s NGN-401 Phase 1/2 trial for Rett syndrome; effects of the removal of staggered dosing in Cohort 1; initiation of new clinical sites for NGN-401 in the UK; expected interactions with the FDA regarding NGN-101; nomination of additional preclinical product candidates; and our expected cash resources and liquidity. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as “may,” “will,” “should,” “would,” “expect,” “anticipate,” “plan,” “likely,” “believe,” “estimate,” “project,” “intend,” “on track,” and other similar expressions or the negative or plural of these words, or other similar expressions that are predictions or indicate future events or prospects, although not all forward-looking statements contain these words. Forward-looking statements are based on current beliefs and assumptions that are subject to risks, uncertainties and assumptions that are difficult to predict with regard to timing, extent, likelihood, and degree of occurrence, which could cause actual results to differ materially from anticipated results and many of which are outside of Neurogene’s control. Such risks, uncertainties and assumptions include, among other things: risks related to the timing and success of enrolling patients in the expanded cohort of our Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome; the expected timing and results of dosing of patients in our clinical trials, including NGN-401; the potential that we may not be able to expand our Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome into the UK based on a variety of factors, including but not limited to any decisions of regulatory authorities, costs of expanding the trial in the UK, the availability of suitable clinical test sites, and the ability to enroll patients in the UK or other reasons; the potential for negative impacts to patients resulting from using a higher dose of NGN-401 in Cohort 2 of the Phase 1/2 clinical trial for the treatment of Rett syndrome; the risk that we may not be able to report our data on the predicted timeline; our limited operating history; the risk that we may not be able to raise adequate additional capital to finance our operations, complete our clinical trials and commercialize our products; risks related to our ability to obtain regulatory approval for, and ultimately commercialize, our product candidates, including NGN-401; risks related to the outcome of non-clinical testing and early clinical trials for our product candidates, including the ability of those trials to satisfy relevant governmental or regulatory requirements; risks related to our limited experience in designing clinical trials and lack of experience in conducting clinical trials; and other risks and uncertainties identified under the heading “Risk Factors” included in our Annual Report on Form 10-K for the year ended December 31, 2023, filed with the Securities and

Exchange Commission (“SEC”) on March 18, 2024, and other filings that the Company has made and may make with the SEC in the future. Nothing in this communication should be regarded as a representation by any person that the forward-looking statements set forth herein will be achieved or that the contemplated results of any such forward-looking statements will be achieved. Forward-looking statements in this communication speak only as of the day they are made and are qualified in their entirety by reference to the cautionary statements herein. Except as required by applicable law, Neurogene undertakes no obligation to revise or update any forward-looking statement, or to make any other forward-looking statements, whether as a result of new information, future events or otherwise.

This communication contains hyperlinks to information that is not deemed to be incorporated by reference into this communication.

- Financial Tables Follow -

**Neurogene Inc.
Condensed Consolidated Balance Sheet Data
(In thousands of U.S. dollars)**

	December 31, 2023	December 31, 2022
Assets		
Cash and cash equivalents	\$ 148,210	\$ 82,021
Other current assets	52,138	2,698
Non-current assets	22,225	24,546
Total assets	\$ 222,573	\$ 109,265
Liabilities		
Current liabilities	\$ 22,973	\$ 6,651
Non-current liabilities	13,576	3,987
Total liabilities	36,549	10,638
Total convertible preferred stock	—	244,366
Stockholders' equity (deficit)	186,024	(145,739)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	\$ 222,573	\$ 109,265

**Neurogene Inc.
Condensed Consolidated Statements of Operations
(In thousands of U.S. dollars, 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, net	2,911	1,328
Bargain purchase gain	16,355	—
Net loss	\$ (36,317)	\$ (55,189)

	Pre-Merger		Post-Merger	Pre-Merger	
	(a)	(b)	(c)	(a)	(b)
Per share information ⁽¹⁾ :					
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.

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