Neurogene Reports Third Quarter 2024 Financial Results and Highlights Recent Updates

November 18, 2024

Announced positive interim clinical data with 1E15 vg dose of NGN-401 gene therapy trial for Rett syndrome

Neurogene to advance NGN-401 at 1E15 vg dose

Expects to provide an update on registrational trial design in first half of 2025

Strong cash position provides runway into the second half of 2027

NEW YORK--(BUSINESS WIRE)--Nov. 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 third quarter 2024 financial results and highlighted recent corporate updates.

“The recently announced interim data from participants dosed with 1E15 vg in the NGN-401 clinical trial showed consistent improvements across multiple domains that define Rett syndrome, in contrast to what is expected based on the natural history of the disease,” stated Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. “We have observed girls gaining complex skills rarely ever learned in this population, as well as skills that were once present and lost during the phase of developmental regression. The favorable safety and efficacy data for NGN-401 at the 1E15 vg dose demonstrate its potential to have a meaningful impact on the disease course of Rett syndrome. We will continue to engage with the FDA on the planning for a future registrational clinical trial and anticipate providing an update on the trial design in the first half of 2025.”

Third Quarter 2024 and Recent Highlights, and Anticipated Milestones

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

NGN-401 is currently being evaluated in an ongoing, open-label Phase 1/2 trial. Key updates include:

- Announced today an update to the NGN-401 clinical trial
  - Following a treatment-related serious adverse event (SAE) in the third participant dosed with 3E15 vg, Neurogene paused further use of the 3E15 vg dose and does not plan to enroll any further participants at the 3E15 vg dose
  - The U.S. Food and Drug Administration (FDA) has completed a review of the safety data for NGN-401 and has allowed Neurogene to proceed with the Phase 1/2 trial using the 1E15 vg dose
  - Neurogene’s prior guidance of completing enrollment this quarter in the 1E15 vg dose cohort was updated as a result of the need to revise the protocol to remove the 3E15 vg dose; Neurogene expects to resume dosing once the revisions to the protocol are completed
- Previously announced positive interim clinical data from the participants dosed with 1E15 vg:
  - The first four participants showed consistent, concordant improvements across key Rett syndrome scales; all achieved clinically meaningful rating of “much improved” on the Clinical Global Impression-Improvement (CGI-I) scale and improved by 28 to 52 percent on the Rett Syndrome Behavioral Questionnaire (RSBQ)
  - Participants achieved meaningful gains of function and developmental milestones in the core clinical domains of Rett syndrome – hand function/fine motor, communication/language, and ambulation/gross motor; these improvements were achieved despite heterogeneous clinical presentation at baseline, and not expected based on the natural history of Rett syndrome
  - NGN-401 was well-tolerated with a favorable safety profile in the first five participants who received the 1E15 vg dose; all treatment-related adverse events (AEs) in these participants were Grade 1 (mild)
- Gained alignment with the FDA on CMC scale-up plans to support commercial launch and potency assay strategy for the program
- Plans to provide an update on registrational trial design in the first half of 2025
- Expects to announce additional interim Phase 1/2 clinical data in the second half of 2025

CLN5 Batten Disease Update

Neurogene completed enrollment (n=6) in the NGN-101 gene therapy for CLN5 Batten disease dose-escalation Phase 1/2 trial in 3Q:24. Key updates include:

- Announced that the Company does not expect to move forward with the NGN-101 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 FDA
- To support a streamlined pathway, Neurogene submitted a Regenerative Medicine Advance Therapy (RMAT) application to the FDA; despite the Company's belief that the application met the standard of preliminary clinical evidence required to obtain an RMAT designation, the RMAT application was denied
- Neurogene is currently evaluating options for the program

Additional Corporate Updates

- Executed an oversubscribed private placement of approximately $200 million in gross proceeds to Neurogene with participation from top-tier healthcare funds, extending cash runway into the second half of 2027
    - Post financing, there are approximately 21.0 million shares of common stock outstanding (assuming the exercise in full of all pre-funded warrants)
- Expects to advance an additional product candidate into the clinic in 2025

Upcoming Events

- Stifel 2024 Healthcare Conference: Management will participate in a fireside chat at 12:40 p.m. ET on November 19 and participate in 1x1 meetings
- British Paediatric Neurology Association Annual Meeting: Encore oral poster presentation of NGN-401 interim clinical data at 11:40 a.m. GMT on January 10, 2025

Third Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and investments as of September 30, 2024 were $139.0 million. The Company continues to expect current cash, cash equivalents and marketable securities, together with an estimated $189.5 million in net proceeds from the private placement, to fund operations into the second half of 2027.
- **Research & Development ("R&D") Expenses:** R&D expenses were $16.3 million for the three months ended September 30, 2024 compared to $11.6 million for the three months ended September 30, 2023. The increase in R&D expenses was primarily driven by an increase in NGN-401 clinical trial costs, increased preclinical costs related to the Company's early discovery programs, and an increase in compensation and benefits expenses due to an increase in R&D headcount.
- **General & Administrative ("G&A") Expenses:** G&A expenses were $5.9 million for the three months ended September 30, 2024 compared to $3.6 million for the three months ended September 30, 2023. The increase in G&A expenses was primarily driven by an increase in employee-related expenses due to an increase in headcount, professional fees, rent, and other corporate-related expenses and market research costs.
- **Net Loss:** Net loss was $20.2 million for the three months ended September 30, 2024 compared to net loss of $14.6 million for the three months ended September 30, 2023.

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, tolerability and efficacy of NGN-401; trial designs, clinical development plans and timing for NGN-401, including anticipated timing of additional clinical trial results from the Company's NGN-401 Phase 1/2 trial for Rett syndrome; expectations regarding a pivotal trial for NGN-401 and expected interactions with the FDA; plans regarding the scale-up of CMC activities in anticipation of a pivotal trial of NGN-401; any potential alternatives for the future development of NGN-101; nomination of additional preclinical product candidates; and The Company's 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:; the expected timing and results of dosing of patients in our Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome; the potential for negative impacts to the Company's Phase 1/2 clinical trial as a result of the previously announced SAE in a patient in the high-dose Cohort 2 of the trial; the risk that the Company may not be able to report our clinical trial data on the predicted timeline; risks related to the Company's ability to obtain regulatory approval for, and ultimately commercialize, our product candidates, including NGN-401; and other risks and uncertainties identified under the heading "Risk Factors" included in Neurogene's 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, or its Quarterly Report on Form 10-Q for the quarter ended June 30, 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 Sheets
(In thousands of U.S. dollars)

	September 30, 2024	December 31, 2023
Assets		
Cash and cash equivalents	$ 66,633	$ 148,210
Other current assets	77,014	52,138
Non-current assets	20,473	22,225
Total assets	$ 164,120	$ 222,573
Liabilities		
Current liabilities	15,800	22,973
Non-current liabilities	10,906	13,576
Total liabilities	26,706	36,549
Stockholders' equity	137,414	186,024
Total liabilities and stockholders' equity	$ 164,120	$ 222,573

Neurogene Inc.
Condensed Consolidated Statements of Operations
(In thousands of U.S. dollars, except share information)

	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, net	1,963	642	5,463	2,150
Net loss	$ (20,217)	$ (14,577)	$ (55,630)	$ (38,700)
Per share information:				
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

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