Neurogene Reports Fourth Quarter and Full Year 2024 Financial Results and Highlights Recent Updates

March 24, 2025

Remains on track to provide regulatory update on registrational trial plans with NGN-401 for Rett syndrome in the first half of 2025

Continues to expect to share additional interim clinical data from NGN-401 Phase 1/2 trial in the second half of 2025

Received PRIME designation for NGN-401 from the European Medicines Agency

Cash runway into the second half of 2027

NEW YORK--(BUSINESS WIRE)--Mar. 24, 2025-- 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 2024 financial results and highlighted recent corporate updates.

“We believe 2024 was a year of significant progress for our NGN-401 Phase 1/2 trial in Rett syndrome, as we reported encouraging interim efficacy data and NGN-401 was generally well-tolerated at the 1E15 vg dose in a pediatric cohort,” said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. “We remain on track to provide an update on the registrational trial plans in the first half of 2025, and as we look ahead to the second half of 2025, we plan to share additional interim clinical data from the Phase 1/2 trial, including from our 11 years and older cohort.”

Fourth 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 open-label Phase 1/2 trial. Key updates include:

- Protocol amended and submitted to U.S. Food and Drug Administration (FDA) with the 1E15 vector genomes (vg) dose for all future participants in the trial, and enrollment is ongoing; 1E15 vg is the dose Neurogene expects to evaluate in a future registrational trial of NGN-401 based on positive interim efficacy results presented in November 2024
- Expanded the age range in the trial to evaluate NGN-401 in a broader population, including ages ≥ 11 years in the older pilot cohort (n=3), from the previous design of ≥ 16 years; the pilot cohort is in addition to the 4 to 10 years cohort (n=8)
- Previously shared the Company gained alignment with the U.S. Food and Drug Administration (FDA) on chemistry, manufacturing and controls (CMC) scale-up plans to support NGN-401 potential commercial launch and potency assay strategy for the program
- Remains on track to provide a regulatory update on registrational trial plans in the first half of 2025
- Continues to expect to report additional interim clinical data from the Phase 1/2 trial in the second half of 2025, including additional participants expected to be dosed during the first half of the year
- Announced today it has received Priority Medicines (PRIME) designation by the European Medicines Agency (EMA); medicines are eligible for PRIME if they demonstrate the potential to address an unmet medical need by showing a meaningful improvement of clinical outcomes

Fourth Quarter and Full Year 2024 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities as of December 31, 2024 were $312.4 million and are expected to provide runway into the second half of 2027, which would allow for the completion of enrollment of a future registrational trial for NGN-401 for Rett syndrome, CMC scale-up to support NGN-401 registrational activities and further development of Neurogene’s EXACT™ gene therapy pipeline.
- **Research & Development (R&D) Expenses:** R&D expenses were $15.3 million and $60.9 million for the three and twelve months ended December 31, 2024, respectively, compared to $12.2 million and $44.4 million for the three and twelve months ended December 31, 2023, respectively. The increase in R&D for the twelve months ended December 31, 2024 was primarily driven by an increase in Rett syndrome clinical trial costs, employee-related expenses due to an increase in headcount, and preclinical costs related to Neurogene’s EXACT gene therapy pipeline.
- **General & Administrative (G&A) Expenses:** G&A expenses were $6.2 million and $22.6 million for the three and twelve months ended December 31, 2024, respectively, compared to $2.5 million and $11.2 million for the three and twelve months ended December 31, 2023, respectively. The increase in G&A expenses for the twelve months ended December 31, 2024 was primarily driven by an increase in employee-related expenses due to an increase in headcount, professional fees, and office-related and other corporate expenses.
- **Net Income and Net Loss:** Net loss was $19.5 million for the three months ended December 31, 2024, and $75.1 million for the twelve months ended December 31, 2024, respectively, compared to net income of $2.4 million and net loss of $36.3 million for the three and twelve months ended December 31, 2023, respectively. Net income for the twelve months ended December 31, 2023 included a one-time $16.4 million bargain purchase gain related to the reverse merger.

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 are made as of the date of this press release. Neurogene does not undertake any obligation to make any updates to these statements to reflect events that occur or circumstances that arise after the date of this press release, except as may be required under applicable U.S. securities law.

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 additional updates for a registrational trial of NGN-401 for Rett syndrome; expected timing for additional interim data from the Company's NGN-401 Phase 1/2 trial for Rett Syndrome, expected future interactions with or positions of the FDA; potential benefits of receiving PRIME designation from the EMA; and the time period over which existing cash resources may be sufficient to fund the Company's operations. 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 Company's Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome; the expected timing and results of dosing of patients in the NGN-401 clinical trial; the potential for negative impacts to participants in the Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome; the risk that the Company may not be able to report data on the predicted timeline; risks related to the Company's ability to obtain regulatory approval for, and ultimately commercialize, its product candidates, including NGN-401; the risk that achievement of PRIME designation for NGN-401 may not result in the anticipated benefits; 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, 2024, filed with the Securities and Exchange Commission ("SEC") on March 17, 2025, 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, 2024	December 31, 2023
Assets		
Cash and cash equivalents	$ 136,586	$ 148,210
Short-term Investments	175,819	48,947
Other current assets	3,518	3,191
Non-current assets	19,807	22,225
Total assets	$ 335,730	$ 222,573
Liabilities		
Current liabilities	15,157	22,973
Non-current liabilities	10,198	13,576
Total liabilities	25,355	36,549
Stockholders' equity	310,375	186,024
Total liabilities and stockholders' equity	$ 335,730	$ 222,573

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

	Year Ended December 31,	
	2024	2023
Revenue under licensing agreements	$ 925	$ —
Operating expenses:		
Research and development expenses	60,917	44,394

General and administrative expenses	22,613	11,189
Total operating expenses	83,530	55,583
Loss from operations	(82,605)	(55,583)
Other income, net	7,461	2,911
Bargain purchase gain	—	16,355
Net loss	$ (75,144)	$ (36,317)

		Pre-Merger		Post-Merger
		(a)	(b)	(c)
Per share information (1):				
Net income (loss) per share, basic	$ (4,28)	$ (117,28)	$ —	$ (27,76)
Weighted-average shares outstanding used in computing net income (loss) per share, basic	17,567,082	426,097	—	491,867
Net income (loss) per share, diluted	$ (4,28)	$ (117,28)	$ —	$ 2,93
Weighted-average shares of common stock outstanding, basic and diluted	17,567,082	426,097	—	4,656,947

(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.

(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.

(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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