



Neurogene Reports First Quarter 2025 Financial Results and Highlights Recent Updates

May 9, 2025

On track to provide registrational trial plan update for NGN-401 gene therapy for Rett syndrome in the first half of 2025

Continues to plan to provide clinical data update from ongoing NGN-401 Phase 1/2 trial in the second half of 2025

Cash runway into the second half of 2027

NEW YORK--(BUSINESS WIRE)--May 9, 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 first quarter 2025 financial results and highlighted recent corporate updates.

"We have made significant progress in planning for our future registrational trial with NGN-401 gene therapy for Rett syndrome and expect to provide an update on our plans during the first half of this year," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "As we work to finalize our proposed trial design to differentiate and position the product candidate as potentially best-in-class for future commercialization, we appreciate the continued collaboration with the FDA. We also advanced the Phase 1/2 trial of NGN-401, and we look forward to sharing updated interim clinical data from the trial in the second half of 2025."

First Quarter 2025 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:

- Remains on track to provide a regulatory update on registrational trial plans in the first half of 2025
- Enrolling additional participants in the Phase 1/2 trial, and continues to expect to report additional interim clinical data from the trial in the second half of 2025; the trial includes a pediatric cohort of females aged 4-10 years old (N=8) and an older cohort of females aged 11 years and older (N=3)
- Announced a [peer-reviewed publication](#) in *Science Translational Medicine* providing proof-of-concept for Neurogene's EXACT™ transgene regulation technology and showing promising tolerability and efficacy in nonclinical studies compared to conventional gene therapy; the publication details EXACT's ability to regulate transgene expression to a tolerable and therapeutic level in non-clinical studies, which led to the development of NGN-401

Upcoming Events

- American Society of Gene and Cell Therapy (ASGCT) Annual Meeting:
 - Fireside chat: Reviving Hope in Deprioritized Cell and Gene Therapy Programs at 3:45 p.m. CT on May 14
 - Panel presentation: FDA's START Pilot Program in Action: Insights from Year One at 3:45 p.m. CT on May 15
 - Oral presentation: Hemophagocytic Lymphohistiocytosis (HLH)/ Hyperinflammatory Syndrome Following High Dose AAV9 Therapy at 3:00 p.m. CT on May 16
- H.C. Wainwright 3rd Annual BioConnect Investor Conference: Management will participate in a fireside chat at 9:30 a.m. ET on May 20 and participate in 1x1 meetings
- Goldman Sachs 46th Annual Global Healthcare Conference: Management will participate in a corporate presentation at 8:00 a.m. ET on June 11 and participate in 1x1 meetings

First Quarter 2025 Financial Results

- **Cash, Cash Equivalents and Short-Term Investments:** Cash, cash equivalents and short-term investments as of March 31, 2025 were \$292.6 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, chemistry, manufacturing and controls (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 \$17.8 million for the three months ended March 31, 2025 compared to \$13.5 million for the three months ended March 31, 2024. The increase in R&D expenses for the three months ended March 31, 2025 was primarily driven by an increase in Rett syndrome clinical trial costs and employee-

related expenses due to an increase in headcount.

- **General & Administrative (G&A) Expenses:** G&A expenses were \$8.2 million for the three months ended March 31, 2025 compared to \$5.2 million for the three months ended March 31, 2024. The increase in G&A expenses for the three months ended March 31, 2025 was primarily driven by an increase in employee-related expenses due to an increase in headcount and other corporate expenses.
- **Net Loss:** Net loss was \$22.6 million for the three months ended March 31, 2025 compared to \$16.9 million for the three months ended March 31, 2024.

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; 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; 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; and other risks and uncertainties identified under the heading "Risk Factors" included in Neurogene's Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, filed with the Securities and Exchange Commission ("SEC") on May 9, 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)

	March 31, 2025	December 31, 2024
Assets		
Cash and cash equivalents	\$ 70,771	\$ 136,586
Short-term investments	221,831	175,819

Other current assets	3,796	3,518
Non-current assets	18,903	19,807
Total assets	\$ 315,301	\$ 335,730
Liabilities		
Current liabilities	14,094	15,157
Non-current liabilities	9,412	10,198
Total liabilities	23,506	25,355
Stockholders' equity	291,795	310,375
Total liabilities and stockholders' equity	\$ 315,301	\$ 335,730

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

	Three Months Ended March 31,	
	2025	2024
Operating expenses:		
Research and development expenses	17,760	13,541
General and administrative expenses	8,159	5,238
Total operating expenses	25,919	18,779
Loss from operations	(25,919)	(18,779)
Other income, net	3,272	1,858
Net loss	\$ (22,647)	\$ (16,921)
Per share information:		
Net loss per share, basic and diluted	\$ (1.08)	\$ (1.00)
Weighted-average shares of common stock outstanding, basic and diluted	20,996,287	16,903,735

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