Neurogene Reports First Quarter 2026 Financial Results and Highlights Recent Updates

May 12, 2026

Dosed ~90% of participants in Embolden™ registrational trial of NGN-401 for Rett syndrome; on track to complete dosing in the second quarter of 2026

NGN-401 has been generally well-tolerated, with no cases of HLH at the 1E15 vg dose; additional interim Phase 1/2 data expected mid-2026

Presentation at ASGCT Meeting highlighted therapeutic rationale for ICV delivery in CNS-targeted gene therapy, including NGN-401

Strong cash position provides runway through first quarter of 2028

NEW YORK--(BUSINESS WIRE)--May 12, 2026-- 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 2026 financial results and highlighted recent corporate updates.

"The significant unmet need in Rett syndrome and strong interest among physicians and caregivers continue to drive momentum for NGN-401, with approximately 90% of participants dosed in the Embolden™ registrational trial," stated Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "We continue to be encouraged that NGN-401 at the 1E15 vg dose has been generally well-tolerated. We are laser-focused on completing dosing in Embolden in the coming weeks and look forward to providing an update on this important clinical development milestone. In parallel, we are continuing to strengthen the organization and execute on early commercial-readiness activities to support a successful potential launch of NGN-401."

First Quarter 2026 and Recent Highlights

NGN-401 Gene Therapy for the Treatment of Rett Syndrome

- Announced today that ~90% of participants have been dosed in the Embolden registrational trial, and the Company is on track to complete dosing in the second quarter of 2026
- NGN-401 at the 1E15 vg dose has been generally well-tolerated, with no cases of hemophagocytic lymphohistiocytosis (HLH) in the Phase 1/2 trial or Embolden as of May 11, 2026
- Presented on the therapeutic rationale for intracerebroventricular (ICV) administration of central nervous system (CNS)-targeted gene therapy, including NGN-401, at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting
    - Presentation highlighted the biological need to deliver gene therapy to the key regions of the brain underlying disease, supported by preclinical data demonstrating broader CNS biodistribution to brain regions central to Rett syndrome pathophysiology, as compared with intrathecal lumbar (IT-L) administration

Additional Corporate Updates

- Strengthened the leadership team with the appointment of Christy Shafer as Chief Commercial Officer and Christine Mikail, J.D., President and CFO of Neurogene, to the Board of Directors
    - Ms. Shafer brings more than 20 years of industry experience building and leading high-performing commercial organizations and launching therapies for rare neurological diseases
    - Ms. Mikail brings to the Board deep expertise in strategic transactions, capital formation, and corporate development, with a track record of building and positioning companies for long-term value creation

Key Anticipated NGN-401 Milestones in 2026

- Complete dosing of participants in the Embolden registrational trial in the second quarter of 2026
- Present updated interim safety and efficacy data on the pediatric and adolescent/adult cohorts from the Phase 1/2 trial, including at least 12 months of follow-up for all 10 participants, in mid-2026
- Initiate Process Performance Qualification (PPQ) campaign in mid-2026
- Continue early commercial-readiness activities

Upcoming Events

- H.C. Wainwright 4th Annual BioConnect Investor Conference: Management will participate in a fireside chat at 11:30 a.m. ET on May 19 and participate in investor meetings
- Goldman Sachs 47th Annual Global Healthcare Conference: Management will participate in a fireside chat at 2:40 p.m. ET on June 8 and participate in investor meetings

First Quarter 2026 Financial Results

- **Cash, Cash Equivalents and Short-Term Investments**: Cash, cash equivalents and short-term investments as of March 31, 2026 were $243.2 million and are expected to fund planned operations through the first quarter of 2028.
- **Research & Development (R&D) Expenses**: R&D expenses were $25.2 million for the three months ended March 31, 2026, compared to $17.8 million for the three months ended March 31, 2025. The increase in R&D expenses for the three months ended March 31, 2026 was primarily driven by an increase in Rett syndrome clinical trial costs of NGN-401, chemistry, manufacturing and controls (CMC) costs to support NGN-401 and employee-related expenses due to an increase in R&D headcount. The increase was partially offset by decreases in spending on the CLN5 Batten disease program and early discovery.
- **General & Administrative (G&A) Expenses**: G&A expenses were $8.2 million for the three months ended March 31, 2026 and the three months ended March 31, 2025. Higher costs related to corporate and pre-commercial activities, along with increased employee-related expenses and professional fees, were offset by a decrease in non-cash stock-based compensation expense.
- **Net Loss**: Net loss was $30.9 million for the three months ended March 31, 2026, compared to $22.6 million for the three months ended March 31, 2025.

About Neurogene
Neurogene (NASDAQ: NGNE) is a clinical-stage biotechnology company focused on developing life-changing genetic medicines for people and their families impacted by devastating neurological diseases. The Company is using a biology-first approach paired with optimized delivery to develop purpose-built genetic medicines, including programs powered by its novel and proprietary EXACT™ transgene regulation technology. Neurogene is advancing its lead gene therapy program, NGN-401, as a potential best-in-class, one-time treatment for Rett syndrome. For more information, visit neurogene.com or follow on LinkedIn.

About NGN-401
NGN-401 is an investigational AAV9 gene therapy in late-stage clinical development as a potential best-in-class, one-time treatment for Rett syndrome. It is the only clinical candidate to deliver the full-length human *MECP2* gene and includes Neurogene's EXACT™ transgene regulation technology, which is designed to deliver consistent, tightly controlled MeCP2 protein expression on a cell-by-cell basis. NGN-401 is delivered through intracerebroventricular administration to achieve the broadest targeting directly to the brain and nervous system based on nonclinical biodistribution data. NGN-401 is being evaluated in the Embolden™ registrational clinical trial. Interim data from the Phase 1/2 trial (as of October 30, 2025) have shown that participants experienced multidomain, durable gains with continued skill acquisition observed over time, and NGN-401 at the 1E15 vg dose has been generally well-tolerated. NGN-401 has received Breakthrough Therapy, Regenerative Medicine Advanced Therapy, Fast Track, Orphan Drug and Rare Pediatric Disease designations and selection for the START Pilot Program from the U.S. Food and Drug Administration, Advanced Therapy Medicinal Product, Orphan and Priority Medicines designations from the European Medicines Agency and Innovative Licensing and Application Pathway designation from the United Kingdom Medicines and Healthcare products Regulatory Agency.

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; the applicability of reported interim results from the NGN-401 Phase 1/2 clinical trial to other participants or potential participants, including adolescent or adult patients; the potential for NGN-401 to be a best-in-class gene therapy for Rett syndrome; trial designs and clinical development plans for the Company's Embolden registrational clinical trial of NGN-401 for Rett syndrome, including timing of anticipated dosing and completion of participant dosing in the Company's clinical trial and the expected timeline of its PPQ activities for its CMC requirements; the response rate, expected durability and deepening of clinical data results from our NGN-401 clinical trial; the potential for future approval for commercialization of NGN-401 as a treatment for Rett syndrome; commercial launch readiness for NGN-401; expected timing for release of additional data from the Company's Phase 1/2 clinical trial of NGN-401; the potential for success of the Embolden registrational clinical trial of NGN-401 for Rett syndrome; the clinical benefit of delivering NGN-401 via ICV administration; expected future interactions with or positions of the FDA, including the timing and outcome of any such interactions and anticipated benefits of any regulatory designation for NGN-401, including the FDA's Breakthrough Therapy designation, Rare Pediatric Disease designation, RMAT designation and participation in the FDA's START program; 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: 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; risks related to timing of completing enrollment in the Embolden trial of NGN-401 for Rett syndrome; 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, 2025, filed with the Securities and Exchange Commission (“SEC”) on March 24, 2026, the Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, filed with the SEC on May 12, 2026, 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.
Unaudited Condensed Consolidated Balance Sheet Data
(In thousands of U.S. dollars)

	March 31, 2026	December 31, 2025
Assets		
Cash and cash equivalents	$ 124,161	$ 103,845
Short-term investments	119,021	165,168
Other current assets	3,565	2,757
Non-current assets	16,394	16,834
Total assets	$ 263,141	$ 288,604
Liabilities		
Current liabilities	$ 18,866	$ 16,411
Non-current liabilities	6,492	7,306
Total liabilities	25,358	23,717
Stockholders' equity	237,783	264,887
Total liabilities and stockholders' equity	$ 263,141	$ 288,604

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

	Three Months Ended March 31,	
	2026	2025
Operating expenses:		
Research and development expenses	$ 25,150	$ 17,760
General and administrative expenses	8,199	8,159
Total operating expenses	33,349	25,919
Loss from operations	(33,349)	(25,919)
Other income, net	2,415	3,272
Net loss	$ (30,934)	$ (22,647)
Per share information:		
Net loss per share, basic and diluted	$ (1.39)	$ (1.08)

Weighted-average shares of common stock outstanding, basic and diluted	22,305,734	20,996,287

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