Neurogene Reports Full Year 2025 Financial Results and Highlights Recent Updates

March 24, 2026

100% of participants enrolled in Embolden™ registrational trial and >50% of participants dosed; on track to complete dosing in second quarter of 2026

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

FDA Breakthrough Therapy designation granted to NGN-401 based on positive interim safety and efficacy data from Phase 1/2 trial

PPQ campaign planned to begin mid-2026, supporting BLA readiness

Strong cash position provides runway through first quarter of 2028

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

“We continue to demonstrate strong execution, with completion of enrollment in the Embolden registrational trial, dosing more than half of participants, and receipt of Breakthrough Therapy designation for NGN-401,” stated Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. “NGN-401 continued to be generally well-tolerated at the 1E15 vg dose, and we remain on track to complete dosing in the second quarter of 2026. With PPQ planned to begin mid-year and early commercial-readiness activities well underway, we believe NGN-401 is increasingly de-risked as we advance toward a BLA submission and potential launch of NGN-401.”

Recent Corporate and NGN-401 Program Highlights

NGN-401 Gene Therapy for the Treatment of Rett Syndrome

- Announced today 100% of participants are enrolled in the Embolden™ registrational trial and >50% of participants have been dosed, 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 March 23, 2026
- Received Breakthrough Therapy designation for NGN-401 from the U.S. Food and Drug Administration (FDA), which was based on the FDA’s review of interim efficacy and safety data from the Phase 1/2 trial as of the data cutoff date of October 30, 2025, including patient-level data and supporting video documentation
- In preparation for the planned NGN-401 Biologics License Application (BLA) submission, confirmed with the FDA that commercial manufacturing scale is the same as current clinical manufacturing scale, removing the need for comparability studies
- Initiated all 13 U.S. clinical trial sites conducting Embolden, with ongoing activities to convert these sites to commercial sites at launch
- Completed rigorous market payor research confirming strong reimbursement potential of NGN-401
- Reported positive interim data from the pediatric cohort (ages 4-10 cohort) of the Phase 1/2 trial, which demonstrated multidomain, durable gains with continued developmental milestone acquisition as of the data cutoff date of October 30, 2025

Key Anticipated 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 cohort and the adolescent/adult cohort from the Phase 1/2 trial in mid-2026, including at least 12 months of follow-up for all 10 participants
- Initiate Process Performance Qualification (PPQ) campaign in mid-2026
- Initiate additional early commercial-readiness activities

Full Year 2025 Financial Results

- **Cash, Cash Equivalents and Short-Term Investments**: Cash, cash equivalents and short-term investments as of December 31, 2025 were $269.0 million and are expected to fund planned operations through the first quarter of 2028.
- **Research & Development (R&D) Expenses**: R&D expenses were $75.0 million for the twelve months ended December 31, 2025, compared to $60.9 million for the twelve months ended December 31, 2024. The increase in R&D expenses for the twelve months ended December 31, 2025 was primarily driven by an increase in Rett syndrome clinical trial costs and employee-related expenses due to an increase in R&D headcount, and it 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 $28.3 million for the twelve months ended December 31, 2025, compared to $22.6 million for the twelve months ended December 31, 2024. The increase in G&A expenses for the twelve months ended December 31, 2025 was primarily driven by an increase in employee-related expenses due to an increase in non-cash stock-based compensation and headcount.
- **Net Loss**: Net loss was $90.4 million for the twelve months ended December 31, 2025, compared to $75.1 million for the twelve months ended December 31, 2024.

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; 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 our clinical trial and the expected timeline of our process performance qualification (PPQ) activities for our chemistry, manufacturing and controls (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, including the potential for third-party payor approval of reimbursement as well as the potential to convert existing clinical trial sites to commercial sites in the event NGN-401 does receive commercial approval; expected timing for release of additional data from our 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 intracerebroventricular 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2025, filed with the Securities and Exchange Commission ("SEC") on November 13, 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.
Unaudited Condensed Consolidated Balance Sheet Data
(In thousands of U.S. dollars)

	December 31, 2025	December 31, 2024
Assets		
Cash and cash equivalents	$ 103,845	$ 136,586
Short-term investments	165,168	175,819
Other current assets	2,757	3,518
Non-current assets	16,834	19,807
Total assets	$ 288,604	$ 335,730
Liabilities		
Current liabilities	16,411	15,157
Non-current liabilities	7,306	10,198
Total liabilities	23,717	25,355
Stockholders' equity	264,887	310,375
Total liabilities and stockholders' equity	$ 288,604	$ 335,730

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

	Year Ended December 31,	
	2025	2024
Revenue under licensing agreements	$ —	$ 925
Operating expenses:		
Research and development expenses	75,011	60,917
General and administrative expenses	28,317	22,613
Total operating expenses	103,328	83,530
Loss from operations	(103,328)	(82,605)
Other income, net	12,977	7,461
Net loss	$ (90,351)	$ (75,144)
Per share information:		
Net loss per share, basic and diluted	$ (4.24)	$ (4.28)
Weighted-average shares of common stock outstanding, basic and diluted	21,326,283	17,567,082

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