Neurogene Reports Third Quarter 2026 Financial Results and Highlights Recent Updates

November 9, 2026

Topline data from Embolden™ registrational trial anticipated in 2H 2027

Presented new preclinical data highlighting the purposeful design of NGN-401 for Rett syndrome, including rapid onset and durable expression of full-length MECP2 *transgene with single-stranded AAV construct*

NGN-401 at the 1E15 vg dose remains generally well-tolerated (N=35) as of data cutoff date of November 8, 2026

On track to complete PPQ runs by year-end 2026

Strong cash position provides runway into first quarter of 2029

NEW YORK--(BUSINESS WIRE)--Nov. 9, 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 third quarter 2026 financial results and highlighted recent corporate updates.

“During the third quarter, we continued to execute across all aspects of our NGN-401 program as we advance toward potential commercialization,” said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. “With topline data from the Embolden registrational trial expected in the second half of 2027, we are progressing key BLA and commercial-readiness activities, including PPQ runs that we believe remain on track for completion this year. We are also continuing to strengthen our engagement with the Rett syndrome community and the broader medical community to increase awareness and understanding of NGN-401 and the growing body of data supporting its potential as a best-in-class, one-time treatment for Rett syndrome. Together, these efforts are aimed at positioning us for a successful launch, if approved.”

Third Quarter 2026 and Recent Highlights

NGN-401 Gene Therapy for the Treatment of Rett Syndrome

- Anticipate reporting topline data from the Embolden™ registrational trial in the second half of 2027
  - As previously announced, the Embolden trial was overenrolled due to strong demand from the Rett syndrome community. Neurogene elected to include all 25 participants dosed in the primary efficacy analysis. The update to the statistical analysis plan was aligned with the FDA through the START Pilot Program. The corresponding threshold for success to reject the null hypothesis is 9 of 25 participants (36%). All other previously aligned key elements of the trial design remain unchanged.
- Presented an encore of the positive long-term Phase 1/2 data, as of the data cutoff date of June 16, 2026, at the 55th Child Neurology Society Annual Meeting
  - Phase 1/2 data demonstrated developmental milestones were gained in a progressive, stepwise sequence, suggesting a restart of development post-treatment
- Presented data at the European Society of Gene and Cell Therapy 2026 Annual Congress that highlighted the key differentiating design elements of NGN-401 for Rett syndrome:
  - New preclinical data showed rapid onset of action and durable expression of MeCP2 protein following single-stranded AAV (ssAAV) delivery of the NGN-401 construct; the data provided additional evidence that self-complementary AAV does not offer a meaningful advantage in onset of action in the treatment of Rett syndrome
    - NGN-401 was designed to use ssAAV to accommodate the fully functional, full-length *MECP2* transgene rather than a minigene
  - New in vivo evaluation of transgene regulation technologies demonstrated that miRNA-based auto-regulatory technology was not responsive to endogenous MeCP2 levels on a cell-by-cell basis; the data showed expression in both MeCP2-null and MeCP2-expressing neurons
  - An encore presentation supported the therapeutic rationale for the intracerebroventricular (ICV) administration of central nervous system (CNS)-targeted gene therapy, including NGN-401; ICV administration is a standard neurosurgical procedure performed thousands of times per year, and preclinical data demonstrated broader CNS biodistribution to brain regions central to Rett syndrome pathophysiology, as compared with intrathecal lumbar administration

- Partnered with the International Rett Syndrome Foundation to host a webinar focused on educating the Rett syndrome community about NGN-401
- NGN-401 at the 1E15 vg dose has been generally well-tolerated in the Phase 1/2 trial and Embolden (N=35) as of November 8, 2026
- On track to complete Process Performance Qualification (PPQ) runs by the end of 2026 to support the planned Biologics License Application (BLA) submission

Key Anticipated NGN-401 Milestones

- Report topline data from Embolden registrational trial in the second half of 2027
- Complete BLA-enabling PPQ runs by year-end 2026
- Continue commercial-readiness activities

Upcoming Events

- Guggenheim 3rd Annual Healthcare Innovation Conference: Management will participate in a fireside chat at 10:30 a.m. ET on November 10 and participate in investor meetings
- Stifel 2026 Healthcare Conference: Management will participate in a fireside chat at 10:40 a.m. ET on November 11 and participate in investor meetings

Third Quarter 2026 Financial Results

- **Cash, Cash Equivalents and Short-Term Investments**: Cash, cash equivalents and short-term investments as of September 30, 2026 were $359.7 million and are expected to fund planned operations into the first quarter of 2029.
- **Research & Development (R&D) Expenses**: R&D expenses were $23.5 million for the three months ended September 30, 2026, compared to $17.2 million for the three months ended September 30, 2025. The increase in R&D expenses for the three months ended September 30, 2026 was primarily driven by higher costs associated with the development of NGN-401, including Rett syndrome clinical trial activities and chemistry, manufacturing and controls (CMC) activities supporting the NGN-401 program. The increase also reflected higher employee-related expenses due to an increase in R&D headcount and increased non-cash stock-based compensation expenses. The increase was partially offset by lower spending on the CLN5 Batten disease program.
- **General & Administrative (G&A) Expenses**: G&A expenses were $8.9 million for the three months ended September 30, 2026, compared to $6.6 million for the three months ended September 30, 2025. The increase in G&A expenses was primarily driven by higher non-cash stock-based compensation expense, driven by an increase in headcount as well as an increase in performance-based stock unit awards following achievement of specified corporate milestones. Additional increases were driven by corporate and pre-commercial activities, higher employee-related expenses and professional fees.
- **Net Loss**: Net loss was $29.0 million for the three months ended September 30, 2026, compared to $21.0 million for the three months ended September 30, 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. Data from the Phase 1/2 trial (as of June 16, 2026) have shown that participants experienced multidomain, durable gains with continued developmental milestone 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; 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; the expected timing and successful completion of the Company's PPQ campaign and other CMC activities to support a planned BLA submission; 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 topline data from the Company's registrational trial of NGN-401; the potential for success of the Embolden registrational clinical trial of NGN-401 for Rett syndrome; the extent to which preclinical data, including data regarding the onset and durability of transgene expression, transduction efficiency and biodistribution, may be predictive of clinical trial results; clinical benefit of delivering NGN-401 via intracerebroventricular administration; expected future interactions with or positions of the FDA or foreign regulatory authorities, 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 EMA's PRIME designation; plans for a BLA submission for NGN-401; 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 or the Embolden registrational clinical trial of NGN-401 for the treatment of Rett syndrome; the risk that preclinical data or interim clinical results may not be predictive of results of the Embolden registrational trial or of future clinical experience; the risk that the Company may not be able to report data on the predicted timeline; the risk that the Company may not complete its PPQ campaign on the anticipated timeline or at all, or may encounter other manufacturing or CMC challenges; 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 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 June 30, 2026, filed with the SEC on August 11, 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)

	September 30, 2026	December 31, 2025
Assets		
Cash and cash equivalents	$ 78,942	$ 103,845
Short-term investments	280,776	165,168
Other current assets	3,021	2,757
Non-current assets	15,162	16,834
Total assets	$ 377,901	$ 288,604
Liabilities		
Current liabilities	$ 18,163	$ 16,411
Non-current liabilities	4,944	7,306
Total liabilities	23,107	23,717
Stockholders' equity	354,794	264,887
Total liabilities and stockholders' equity	$ 377,901	$ 288,604

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	**2025**	**2026**	**2025**
Operating expenses:				
Research and development expenses	$ 23,522	$ 17,241	$ 74,140	$ 54,372
General and administrative expenses	8,854	6,557	28,255	21,427
Total operating expenses	32,376	23,798	102,395	75,799
Loss from operations	(32,376)	(23,798)	(102,395)	(75,799)
Other income, net	3,353	2,845	7,950	10,183
Net loss	$ (29,023)	$ (20,953)	$ (94,445)	$ (65,616)
Per share information:				
Net loss per share, basic and diluted	$ (1.05)	$ (0.99)	$ (3.90)	$ (3.11)
Weighted-average shares of common stock outstanding, basic and diluted	27,695,698	21,168,431	24,204,232	21,073,996

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