



Neurogene Reports Second Quarter 2026 Financial Results and Highlights Recent Updates

August 11, 2026

Data from Phase 1/2 trial of NGN-401 for Rett syndrome showed 100% of participants improved in CGI-I score and gained ≥ 1 developmental milestone, with an average of 4.7 milestones per participant; no plateau and no milestone loss observed as of data cutoff date of June 16, 2026

Milestones gained in a progressive, stepwise sequence, suggesting a restart of development post-treatment

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

NGN-401 at the 1E15 vg dose remains generally well-tolerated (N=35) as of data cutoff date of August 10, 2026

Initiated PPQ campaign to support BLA submission for NGN-401; completion of PPQ runs expected by end of 2026

Extended cash runway into 1Q 2029 with oversubscribed financing of approximately \$144 million gross proceeds

NEW YORK--(BUSINESS WIRE)--Aug. 11, 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 second quarter 2026 financial results and highlighted recent corporate updates.

"The second quarter and recent months marked important progress across our NGN-401 program, highlighted by positive long-term Phase 1/2 data showing durable, multidomain developmental gains that have not been observed in the natural history of Rett syndrome," stated Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "The clinically meaningful benefit observed across all participants, including 100% of treated participants improving on CGI-I and gaining an average of 4.7 developmental milestones, strengthens our confidence in the program as we advance toward topline Embolden™ registrational data, which are expected in the second half of 2027. With initiation of our PPQ campaign, ongoing preparations for a potential BLA submission and a strengthened balance sheet supporting commercial-readiness activities, we believe we are well positioned to bring NGN-401 to patients and families as quickly as possible, if approved."

Second Quarter 2026 and Recent Highlights

NGN-401 Gene Therapy for the Treatment of Rett Syndrome

- [Reported](#) positive long-term Phase 1/2 data as of the data cutoff date of June 16, 2026, which demonstrated developmental milestones were gained in a progressive, stepwise sequence, suggesting a restart of development post-treatment
 - 100% of participants (N=10) improved on the Clinical Global Impression-Improvement (CGI-I) scale and gained ≥ 1 developmental milestone, consistent with the composite endpoint used to evaluate efficacy in Embolden
 - 47 total developmental milestones were gained for an average of 4.7 per participant; improvement continued across follow-up through 30 months with no plateau or loss of milestone observed
 - 7 of 10 participants gained ≥ 2 developmental milestones and demonstrated improvements across ≥ 2 core Rett syndrome domains; both of these improvements were observed in pediatric and adolescent/adult participants
 - Participants experienced clinically meaningful improvements across additional validated Rett syndrome scales, including the Rett Syndrome Gross Motor Scale (RSGMS) and Rett Syndrome Hand Function Scale (RSHFS) ($p < 0.001$)
- Presented on the Rett Syndrome Natural History Study analysis conducted to assess treatment effect in Embolden, which demonstrated that the likelihood of milestone gains ≥ 3 years of age is rare
- [Completed](#) successful dosing of 25 participants in Embolden; topline data anticipated in the second half of 2027
- Announced today the Process Performance Qualification (PPQ) campaign was initiated in July 2026 to support planned Biologics License Application (BLA) submission; completion of PPQ runs expected by the end of 2026
- NGN-401 at the 1E15 vg dose has been generally well-tolerated in the Phase 1/2 trial and Embolden (N=35) as of August 10, 2026

Additional Corporate Updates

- Executed an oversubscribed public follow-on offering of approximately \$144 million in gross proceeds, including full

exercise of the underwriters' option to purchase additional shares, extending cash runway into the first quarter of 2029

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

Second Quarter 2026 Financial Results

- **Cash, Cash Equivalents and Short-Term Investments:** Cash, cash equivalents and short-term investments as of June 30, 2026 were \$225.4 million. Together with approximately \$134.8 million of net proceeds received from our July 2, 2026 public offering, Neurogene had pro forma cash, cash equivalents and short-term investments of approximately \$360.2 million and expect its current cash resources to fund planned operations into the first quarter of 2029.
- **Research & Development (R&D) Expenses:** R&D expenses were \$25.5 million for the three months ended June 30, 2026, compared to \$19.4 million for the three months ended June 30, 2025. The increase in R&D expenses for the three months ended June 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, primarily due to increased non-cash stock-based compensation expenses. The increase was partially offset by lower spending on the CLN5 Batten disease program and early discovery activities.
- **General & Administrative (G&A) Expenses:** G&A expenses were \$11.2 million for the three months ended June 30, 2026, compared to \$6.7 million for the three months ended June 30, 2025. The increase in G&A expenses was primarily driven by higher non-cash stock-based compensation expense, including a one-time catch-up charge associated with 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 \$34.5 million for the three months ended June 30, 2026, compared to \$22.0 million for the three months ended June 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, including 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 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 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; expected timing and 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 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 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)**

	June 30, 2026	December 31, 2025
Assets		
Cash and cash equivalents	\$ 112,485	\$ 103,845
Short-term investments	112,907	165,168
Other current assets	3,521	2,757
Non-current assets	15,451	16,834
Total assets	\$ 244,364	\$ 288,604
Liabilities		
Current liabilities	\$ 21,505	\$ 16,411
Non-current liabilities	5,726	7,306
Total liabilities	27,231	23,717
Stockholders' equity	217,133	264,887
Total liabilities and stockholders' equity	\$ 244,364	\$ 288,604

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

	Three Months Ended June 30, 2026	2025	Six Months Ended June 30, 2026	2025
Operating expenses:				
Research and development expenses	\$ 25,468	\$ 19,366	\$ 50,618	\$ 37,131
General and administrative expenses	11,202	6,715	19,401	14,869

Total operating expenses	36,670	26,081	70,019	52,000
Loss from operations	(36,670)	(26,081)	(70,019)	(52,000)
Other income, net	2,182	4,065	4,597	7,337
Net loss	<u>\$ (34,488)</u>	<u>\$ (22,016)</u>	<u>\$ (65,422)</u>	<u>\$ (44,663)</u>
Per share information:				
Net loss per share, basic and diluted	<u>\$ (1.53)</u>	<u>\$ (1.05)</u>	<u>\$ (2.92)</u>	<u>\$ (2.12)</u>
Weighted-average shares of common stock outstanding, basic and diluted	22,552,032	21,055,378	22,429,563	21,025,996

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