



Neurogene Reports Second Quarter 2025 Financial Results and Highlights Recent Updates

August 11, 2025

Announced design of Embolden™ registrational clinical trial of NGN-401 gene therapy for Rett syndrome; trial initiation activities underway

Completed dosing in Phase 1/2 NGN-401 trial, and remains on track to report updated clinical efficacy and safety data in the second half of 2025

Cash runway into early 2028

NEW YORK--(BUSINESS WIRE)--Aug. 11, 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 second quarter 2025 financial results and highlighted recent corporate updates.

“In the first half of 2025, we made significant progress in our NGN-401 program for Rett syndrome. We completed dosing of the last five participants in the Phase 1/2 trial and received written agreement from the U.S. FDA on the key elements of the Embolden™ registrational trial, in which we have already begun initiation activities,” stated Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. “We are pleased to be moving forward with Embolden, which was purposefully and rigorously designed to differentiate NGN-401 by evaluating participants as young as three years of age with a primary endpoint that incorporates measures that KOLs, caregivers and payors believe to be clinically meaningful. Our previously announced reallocation of capital provides us runway into early 2028, enabling us to focus our resources on advancement of this potential therapy for the patients and families who urgently need new treatment options.”

Dr. McMinn continued, “In leveraging the continual dialogue under the START program, the FDA encouraged the analysis of the Embolden primary endpoint to remain at 12 months as the basis for full approval, noting that a 6-month endpoint may not be considered clinically meaningful. In further maintaining the rigorous design of the Embolden trial, we are electing to dose the last planned participant from the Phase 1/2 trial as part of the registrational Embolden trial and add one more participant to complete the proposed sample size at 20 patients.”

Second Quarter 2025 and Recent Highlights, and Anticipated Milestones

NGN-401 Gene Therapy for Treatment of Rett Syndrome

- [Received](#) written agreement from the U.S. Food and Drug Administration (FDA) on key elements of the Embolden registrational trial of NGN-401 and refined the sample size to propose 20 participants
- Initiated Embolden clinical trial activities to support the conversion of the Phase 1/2 trial to a registrational trial
- Completed enrollment in the Phase 1/2 trial, with the last 5 additional participants dosed in the first half of 2025
- Remains on track to report updated clinical efficacy and safety data from the Phase 1/2 trial in the second half of 2025
- Presented at scientific conferences the hemophagocytic lymphohistiocytosis (HLH) monitoring and treatment algorithm incorporated into the NGN-401 clinical trial, which has been acknowledged as valuable information by the Rett syndrome and gene therapy communities
 - There has been no evidence of HLH/hyperinflammatory syndrome in any NGN-401 trial participant at the 1E15 vg dose level, as of the date of this press release

Upcoming Events

- Stifel Biotech Summer Summit: Management will participate in a fireside chat at 12:00 p.m. ET on August 12 (not webcast)
- H.C. Wainwright Annual Global Investment Conference: Management will participate in a fireside chat at 2:30 p.m. ET on September 8 and participate in 1x1 meetings

Second Quarter 2025 Financial Results

- **Cash, Cash Equivalents and Short-Term Investments:** Cash, cash equivalents and short-term investments as of June 30, 2025 were \$274.5 million. We currently expect cash, cash equivalents and short-term investments to fund planned operations into early 2028.
- **Research & Development (R&D) Expenses:** R&D expenses were \$19.4 million for the three months June 30, 2025

compared to \$15.7 million for the three months ended June 30, 2024. The increase in R&D expenses for the three months ended June 30, 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.

- **General & Administrative (G&A) Expenses:** G&A expenses were \$6.7 million for the three months ended June 30, 2025 compared to \$5.3 million for the three months ended June 30, 2024. The increase in G&A expenses for the three months ended June 30, 2025 was primarily driven by an increase in employee-related expenses due to an increase in stock-based compensation, headcount and other corporate expenses.
- **Net Loss:** Net loss was \$22.0 million for the three months ended June 30, 2025 compared to \$18.5 million for the three months ended June 30, 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.

About NGN-401

NGN-401 is an investigational AAV9 gene therapy being developed as a one-time treatment for Rett syndrome. It is the first clinical candidate to deliver the full-length human *MECP2* gene under the control of Neurogene's EXACT™ transgene regulation technology. EXACT technology is an important advancement in gene therapy for Rett syndrome, specifically because the disorder requires a treatment approach that enables targeted levels of *MECP2* transgene expression without causing overexpression-related toxic effects associated with conventional gene therapy.

NGN-401 was selected by the U.S. Food and Drug Administration (FDA) for its START Pilot Program and has also received Regenerative Medicine Advance Therapy (RMAT) designation, orphan drug designation, Fast Track designation and rare pediatric designation from the FDA. Neurogene was previously granted an INTERACT meeting with the FDA regarding the EXACT technology. NGN-401 also received Priority Medicines (PRIME) designation, orphan designation and advanced therapy medicinal product designation from the European Medicines Agency (EMA) and the Innovative Licensing and Application Pathway (ILAP) designation from the United Kingdom (UK) Medicines and Healthcare products Regulatory Agency (MHRA).

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 elements of the registrational clinical study trial design subject to final approval of the FDA, such as the proposed number of participants in the Embolden trial, and the timing of the conversion of the NGN-401 Phase 1/2 clinical trial to a registrational clinical trial, anticipated timing of additional updates for the Embolden 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: the expected timing of additional results from 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; risks related to timing of initiating 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 June 30, 2025, filed with the Securities and Exchange Commission ("SEC") on August 11, 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)**

	June 30, 2025	December 31, 2024
Assets		
Cash and cash equivalents	\$ 58,813	\$ 136,586
Short-term investments	215,706	175,819
Other current assets	4,467	3,518
Non-current assets	18,330	19,807
Total assets	\$ 297,316	\$ 335,730
Liabilities		
Current liabilities	15,440	15,157
Non-current liabilities	8,621	10,198
Total liabilities	24,061	25,355
Stockholders' equity	273,255	310,375
Total liabilities and stockholders' equity	\$ 297,316	\$ 335,730

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Revenue under licensing agreements	—	925	—	925
Operating expenses:				
Research and development expenses	19,366	15,744	37,131	29,285
General and administrative expenses	6,715	5,315	14,869	10,553
Total operating expenses	26,081	21,059	52,000	39,838
Loss from operations	(26,081)	(20,134)	(52,000)	(38,913)
Other income, net	4,065	1,642	7,337	3,500
Net loss	\$ (22,016)	\$ (18,492)	\$ (44,663)	\$ (35,413)
Per share information:				
Net loss per share, basic and diluted	\$ (1.05)	\$ (1.09)	\$ (2.12)	\$ (2.09)
Weighted-average shares of common stock outstanding, basic and diluted	21,055,378	16,941,524	21,025,996	16,922,630

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