



## Neurogene Announces Recent Achievements and Anticipated 2026 Key Milestones for NGN-401, a Potential Best-in-Class Gene Therapy for Rett Syndrome

January 12, 2026

*Dosed multiple participants in Embolden™ registrational trial in fourth quarter of 2025*

*Completion of dosing in Embolden expected in second quarter of 2026*

*Plan to present interim data on pediatric and adolescent/adult cohorts from Phase 1/2 trial in mid-2026*

*Early commercial-readiness activities underway*

*Neurogene to present at J.P. Morgan Healthcare Conference on January 14 at 7:30 a.m. PT*

NEW YORK--(BUSINESS WIRE)--Jan. 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 provided an update on recent achievements for NGN-401 gene therapy for the treatment of Rett syndrome and plans for multiple value-creating catalysts anticipated in 2026.

“We are pleased to share that multiple participants have been dosed in our Embolden registrational trial of NGN-401 gene therapy for Rett syndrome, an important step toward completing dosing in the second quarter,” stated Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. “We continue to track the progress of the participants in our Phase 1/2 trial, with plans to report updated interim safety and efficacy data by mid-year, including at least 12 months of data for all participants. As we rapidly advance NGN-401 towards commercialization, we are actively engaging with payors and other key stakeholders while leveraging our internal CMC capabilities for commercial-grade production to ensure future launch readiness. With cash runway expected through the first quarter of 2028, we believe we are well-positioned to advance NGN-401 through key upcoming milestones, including a BLA submission, to achieve its potential as a best-in-class gene therapy for Rett syndrome.”

### **NGN-401 Program Updates and Recent Accomplishments**

- Dosed multiple participants in the Embolden™ registrational trial in the fourth quarter of 2025
- Initiated 13 U.S. clinical trial sites conducting Embolden
- Reported positive interim data from the Phase 1/2 trial evaluating NGN-401 in females with Rett syndrome; data showed durable multidomain improvements across a full spectrum of disease severity
- Completed rigorous market payor research confirming strong reimbursement potential of NGN-401

### **Key Anticipated Milestones in 2026**

- Complete dosing participants in the Embolden registrational trial in the second quarter of 2026
- Present interim safety and efficacy data on the pediatric cohort (ages 4-10; n=8) and the adolescent/adult cohort (ages ≥ 11; n=2) from the Phase 1/2 trial in mid-2026, including at least 12 months of follow-up for all participants
- Initiate additional early commercial-readiness activities

Company management will participate in the 44<sup>th</sup> Annual J.P. Morgan Healthcare Conference with a presentation on Wednesday, January 14 at 7:30 a.m. PT. A webcast of the presentation will be accessible from the Investor Relations section of Neurogene’s [website](#) under events, where a replay of the event will also be available for a limited time.

### **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](http://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 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 participants; the potential for NGN-401 to be a best-in-class gene therapy for Rett syndrome; trial designs and clinical development plans for our Embolden™ registrational clinical trial of NGN-401 for Rett Syndrome, including timing of anticipated dosing and completion of participant dosing in our clinical trial; the response rate, expected durability and deepening of clinical data results from our NGN-401 clinical trials; 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; our anticipated readiness for commercialization of NGN-401 if it receives regulatory approval; expectations related to payer reimbursement for NGN-401 if approved, including estimates related to potential reimbursement rates, the ease of obtaining reimbursement and the sentiments of payers with respect to value placed on certain outcomes and any impact of potentially implementing an outpatient regimen for NGN-401; expected future interactions with or positions of the FDA or foreign regulatory authorities, including the timing and outcome of any such interaction and anticipated benefits of any regulatory designation for our product candidates, including the FDA's RMAT designation, the EMA's PRIME designation and participation in the FDA's START program with respect to NGN-401; the benefits of Neurogene's in-house manufacturing capabilities; and the time period over which our capital resources will be sufficient to fund our 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 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, 2024, filed with the Securities and Exchange Commission (SEC) on March 24, 2025, Neurogene's Quarterly Report on Form 10-Q for the quarter ended September 30, 2025, filed with the 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.

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### Company Contact:

Mike Devine  
Executive Director, Corporate Communications  
[michael.devine@neurogene.com](mailto:michael.devine@neurogene.com)

### Investor Contact:

Melissa Forst  
Argot Partners

[Neurogene@argotpartners.com](mailto:Neurogene@argotpartners.com)

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