Neurogene Reports Positive Long-term Clinical Data from Phase 1/2 Trial of NGN-401 Gene Therapy for Rett Syndrome

June 29, 2026

47 total developmental milestones gained across 10 participants, with durable treatment effect and no plateau or milestone loss through 30 months of follow-up

100% of participants improved in CGI-I score and gained ≥1 developmental milestone, with an average of 4.7 milestones per participant, and all gaining ≥1 developmental milestone in the past 12 months

Developmental milestones were gained in a progressive, stepwise sequence, suggesting a restart of development post-treatment

NGN-401 at the 1E15 vg dose remains generally well-tolerated (N=35) as of data cutoff date of June 16, 2026

Dosing complete in the Embolden™ registrational trial with no treatment-related SAEs or DLTs as of data cutoff date of June 16, 2026, with topline data anticipated in 2H 2027

Company to host investor/analyst webcast today at 8:00 a.m. ET

NEW YORK--(BUSINESS WIRE)--Jun. 29, 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 updated, positive data from its Phase 1/2 clinical trial evaluating NGN-401 gene therapy for the treatment of females with Rett syndrome.

Across 10 participants, 47 total developmental milestones were gained (average of 4.7 per participant), with both pediatric and adolescent/adult participants demonstrating clinical response and continued, progressive improvements through 30 months of follow-up, with no plateau or loss of milestones observed.

“We are encouraged by the long-term Phase 1/2 data across a broad age range and wide spectrum of disease severity, which we believe demonstrate that NGN-401 is driving gain of durable and clinically meaningful developmental milestones, and continued improvements were observed through 30 months of follow-up,” said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. “Importantly, the gains of developmental milestones are not isolated events – they build over time with participants acquiring multiple milestones across the core Rett syndrome functional domains of hand function, gross motor function and communication. This multidomain progression is not observed in the natural history of Rett syndrome, and we believe supports NGN-401’s differentiated profile, driven by its purposeful design and targeted delivery.”

“In typical development, children gain milestones in an organized, cumulative sequence, with each milestone building on the last; in Rett syndrome, that process is stopped by regression,” said Bernhard Suter, M.D., Medical Director of the Blue Bird Circle Rett Center at Texas Children’s Hospital, Associate Professor of Pediatrics and Neurology at Baylor College of Medicine, and principal investigator in the NGN-401 Phase 1/2 clinical trial. “These results show that the participants treated with NGN-401 are now gaining developmental milestones in a manner that suggests restarting developmental progression, something not observed in the natural history of the disease. As a result, participants are gaining greater independence and are more engaged in day-to-day activities with their families.”

Phase 1/2 Clinical Data as of Data Cutoff Date of June 16, 2026 (N=10, follow-up 12-30 months post-treatment)

Efficacy Data

- 100% of participants 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 the Embolden™ registrational trial
- 47 total developmental milestones were gained across participants, averaging 4.7 milestones per participant
- All participants gained ≥1 developmental milestone in the past 12 months
- Participants gained milestones in a progressive, developmentally ordered stepwise sequence, suggesting a restart of developmental progression
- Rapid onset of treatment effect, with median time to first clinical improvement of 2 months post-treatment
- Milestone gains deepened over time, increasing by 95% from 6 to 12 months and 147% from 6 to ≥12 months
- 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

- Continued improvement through 30 months post-dose, with no plateau observed and no milestones lost
- 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) ($p<0.001$)

Safety Data

- NGN-401 at the 1E15 vg dose remains generally well-tolerated
- All treatment-related adverse events have been mild (Grade 1) or moderate (Grade 2) in severity, and the majority are known potential risks of AAV and have resolved or are resolving
- No new treatment-related SAEs reported since last data cutoff date in October 2025
- NGN-401 continues to be generally well-tolerated in Embolden, with no treatment-related SAEs or DLTs as of the data cutoff date of June 16, 2026

Neurogene management and Dr. Suter will discuss these results during a live webcast today, June 29, 2026, at 8:00 a.m. ET. The live webcast presentation will be accessible from the Investor Relations section of the Company's website under Events, where a replay of the event will also be available for a limited time.

These data, as well as a poster presentation on the Rett Syndrome Natural History Study analysis to assess treatment effect in the Embolden trial, will also be presented at the 2026 International Rett Syndrome Foundation (IRSF) Rett Syndrome Scientific Meeting taking place in Prior Lake, Minn., June 29 – July 1, 2026.

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 results from the NGN-401 Phase 1/2 clinical trial to participants in the Embolden registrational trial; 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 response rate, expected durability and deepening of clinical data results from our NGN-401 clinical trial; expected timing and availability for release of topline data from our Embolden clinical trial of NGN-401 for Rett syndrome; the potential for success of the Embolden trial; the clinical benefit of delivering NGN-401 via intracerebroventricular administration; and 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. 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, filed with the Securities and Exchange Commission ("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.

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