Neurogene Announces Successful Completion of Dosing in Embolden™ Registrational Trial of NGN-401 for Rett Syndrome

June 8, 2026

Exceeded target by dosing a total of 25 participants with NGN-401 in registrational trial due to strong demand from Rett syndrome community

NGN-401 has been generally well-tolerated, with no cases of HLH at the 1E15 vg dose; additional interim Phase 1/2 data expected mid-2026

Topline data from Embolden anticipated in 2H 2027

NEW YORK--(BUSINESS WIRE)--Jun. 8, 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 the successful completion of dosing in the Embolden™ registrational trial of NGN-401, an investigational gene therapy designed to be a potential best-in-class, one-time treatment for Rett syndrome. While the initial enrollment target was 20 participants, the statistical analysis plan pre-specified an intent-to-treat (ITT) population of up to 24 participants, providing flexibility to support robust trial execution. Due to strong demand from the Rett syndrome community and to ensure timely completion of dosing, the Company elected to overenroll the trial and dose all eligible participants already in screening for a total of 25.

“We are pleased to have completed dosing in the Embolden trial of NGN-401 within our original timeline while exceeding our enrollment target, reflecting both the significant unmet need and strong demand from the Rett syndrome community,” stated Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. “This milestone brings us one step closer to delivering a potential best-in-class, one-time treatment for Rett syndrome. We are deeply grateful to the participants, their families and the investigators for their trust, partnership and ongoing support of the development of NGN-401. We look forward to sharing topline results from Embolden in the second half of 2027 and advancing NGN-401 towards a planned BLA submission.”

Embolden is a multi-center, single-arm, open-label, baseline-controlled registrational trial designed to evaluate the efficacy, safety and tolerability of a one-time dose of NGN-401 (1E15 vg) in females with Rett syndrome ages three and older. The primary endpoint is a composite of a Clinical Global Impression-Improvement (CGI-I) score of ≤ 3 and a gain from baseline of any one developmental milestone from a pre-specified list.

The primary analysis to support the planned Biologics License Application (BLA) submission is expected to occur after the first 24 participants (pre-specified ITT) have completed 12 months of follow-up, with topline data expected in the second half of 2027. The threshold for success required for the registrational trial is a 33% (or 8 of 24 participants) minimum response rate. Data from the additional participant is expected to supplement the overall safety and durability dataset.

As of June 7, 2026, NGN-401 at the 1E15 vg dose (n=35) continues to be generally well-tolerated. There were no cases of hemophagocytic lymphohistiocytosis (HLH) at this dose level in the Phase 1/2 trial or the Embolden trial.

The Embolden trial builds on positive interim data from the Phase 1/2 trial of NGN-401, which demonstrated multidomain, durable gains with continued developmental milestone acquisitions as of the most recently disclosed safety and efficacy data, with a cutoff date of October 30, 2025.

Neurogene plans to present updated interim safety and efficacy data from the Phase 1/2 trial, including at least 12 months of follow-up for all 10 participants, in mid-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. Interim data from the Phase 1/2 trial (as of October 30, 2025) have shown that participants experienced multidomain, durable gains with continued skill 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 or potential participants, including adolescent or adult patients; 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 expectations to supplement the overall safety and durability set with data from the additional participant; 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; expected timing for release of additional data from Neurogene's Phase 1/2 clinical trial of NGN-401 and topline data from the Company's Embolden registrational trial of NGN-401; the potential for success of the Embolden registrational clinical trial of NGN-401 for Rett syndrome; the timing and potential for success of a BLA submission to the FDA for NGN-401; 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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Media Contact:
Mike Devine
Executive Director, Corporate Communications
michael.devine@neurogene.com

Investor Contact:
Lina Li
Executive Director, Investor Relations
lina.li@neurogene.com

Source: Neurogene Inc.