Neurogene Highlights Data at ASGCT Meeting Supporting Therapeutic Rationale for ICV Delivery Method in CNS-targeted Gene Therapy

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ICV is a commonly used neurosurgical procedure performed across multiple therapeutic settings, including gene therapy

Presentation adds to body of evidence that ICV administration results in broad biodistribution of gene therapy to the brain and nervous system

Use of ICV administration in NGN-401 gene therapy program for Rett syndrome provides potential rationale for the multidomain and durable improvements observed in Phase 1/2 trial

NEW YORK--(BUSINESS WIRE)--May 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, highlighted data at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting supporting the intracerebroventricular (ICV) route of administration as a routine approach for delivering medicines, including gene therapies, to treat central nervous system (CNS) disorders. The presentation further highlights that the ICV procedure is routine, and administration takes approximately 10 minutes; therefore, the clinical safety and monitoring in gene therapy programs is driven primarily by factors related to transgene expression, dose and total viral load rather than the delivery procedure itself.

“AAV-based gene therapies can only be administered once, making delivery to the affected cells one of the most critical components in designing a potential treatment,” said Daniel J. Curry, M.D., Director of Functional Neurosurgery and Epilepsy Surgery at Texas Children's Hospital, Professor of Neurological Surgery at Baylor College of Medicine, and neurosurgeon in the NGN-401 clinical program. “With broader brain biodistribution compared to other delivery routes and a well-established, low-complexity neurosurgical profile, ICV administration is the most compelling delivery route to date for CNS gene therapies such as those for Rett syndrome. The clinical safety and efficacy data observed in the ICV-delivered NGN-401 gene therapy program for Rett syndrome reaffirm the potential for this route of administration to target the areas of the brain and nervous system affected by Rett syndrome.”

The poster, “Gene Therapy Targeting CNS Diseases: ICV Administration as a Growing Standard for Delivery,” provides an overview of ICV delivery of NGN-401 gene therapy for Rett syndrome with findings from a preclinical study as well as an ongoing Phase 1/2 trial in participants with Rett syndrome, which has completed dosing.

- The ICV procedure is utilized by pediatric and adult neurosurgeons tens of thousands of times annually, and the administration of NGN-401 gene therapy takes approximately 10 minutes.[1]
- Compared to intrathecal lumbar (IT-L) delivery of NGN-401, ICV delivery showed greater biodistribution to the key areas of the brain, including the motor cortex, frontal cortex and cerebellum, and CNS that underlie Rett syndrome in preclinical models.[2]
- In the Phase 1/2 trial of NGN-401, all participants who received the 1E15 vg dose in the pediatric cohort (ages 4-10; n=8) showed functional improvements, with a total of 35 developmental milestones gained with no plateau up to 24 months, and NGN-401 and the ICV procedure were generally well-tolerated as of the data cutoff date of October 30, 2025 (n=10).
- NGN-401 is also being administered through ICV in the Embolden™ registrational clinical trial, and investigators have the option to discharge participants one day after the procedure and implement an outpatient gene therapy monitoring protocol.
    - Clinical safety in CNS gene therapy is driven primarily by vector biology, dose and transgene expression rather than the route of administration itself.

The poster is available in the “Publications” section on the Company’s website.

[1]Data on file at Neurogene Inc.

[2]Burstein SR, *et al*. Self-regulating AAV gene therapy for Rett syndrome: preclinical and translational considerations. *ESGCT Annual Congress*. 2025;36(Suppl 1).

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 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 Neurogene'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 trials; the potential for success of the Embolden registrational clinical trial of NGN-401 for Rett Syndrome; the clinical benefit of delivering NGN-401 via ICV administration; and expected or 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 Pilot 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 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, and other filings that Neurogene 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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