



Neurogene Announces Upcoming Presentation on the Therapeutic Rationale for ICV Delivery in CNS-Targeted Gene Therapy

April 28, 2026

Presentation at ASGCT Meeting will feature ICV as a routine neurosurgical procedure that provides broad biodistribution of gene therapy to the brain and nervous system

Safety and efficacy of NGN-401 for Rett syndrome, delivered via ICV administration, to be highlighted

NEW YORK--(BUSINESS WIRE)--Apr. 28, 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 an upcoming poster presentation that will demystify the intracerebroventricular (ICV) route of administration as a well-established, routinely performed neurosurgical procedure and a deliberate delivery strategy designed to maximize efficacy for central nervous system (CNS) gene therapies at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting, taking place May 11-15, 2026.

The presentation will highlight the biological rationale to deliver gene therapy broadly to key regions of the brain, leading to the selection of ICV administration of NGN-401 gene therapy for Rett syndrome. Preclinical data will show that ICV administration resulted in greater biodistribution to brain regions central to Rett syndrome pathophysiology, compared to intrathecal-lumbar administration. The presentation will review that clinical safety in CNS gene therapy is shown to be driven by the product itself, and not the delivery route, with ICV administration showing a generally well-tolerated profile.

“Our selection of ICV delivery for NGN-401 was informed by robust preclinical data that showed broader brain and nervous system biodistribution compared to IT-L, critical for a CNS-mediated disease such as Rett syndrome,” stated Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. “ICV administration is a well-established neurosurgical approach that has been used across multiple therapeutic areas and represents a deliberate choice to enable effective gene delivery. We believe the data we have reported from our ongoing Phase 1/2 trial reinforce our choice of ICV delivery, with participants demonstrating durable, multidomain developmental milestone gains over time and a safety profile at the 1E15 vg dose that supports continued advancement of NGN-401 in our Embolden™ registrational trial.”

Poster Presentation Details

- **Title:** Gene Therapy Targeting CNS Diseases: ICV Administration as a Growing Standard for Delivery
- **Date:** Tuesday, May 12
- **Presentation Time:** 5-6:30 p.m. ET
- **Presenter:** 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
- **Location:** MCEC Exhibit and Poster Hall

The abstract is available on ASGCT's [website](#).

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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