



Neurogene Announces Upcoming Oral Presentation on Monitoring and Treatment to Reverse Rare Complication of High Dose Gene Therapy at the ASGCT Annual Meeting

April 28, 2025

Presentation will highlight examples of successful surveillance and treatment of HLH in the context of gene therapy

NEW YORK--(BUSINESS WIRE)--Apr. 28, 2025-- 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 oral presentation and participation in a fireside chat and panel discussion on critical gene therapy topics during the American Society for Gene and Cell Therapy (ASGCT) 28th Annual Meeting on May 13-17, 2025 in New Orleans. The oral presentation will include approaches for monitoring and managing hemophagocytic lymphohistiocytosis (HLH), a rare, severe hyperinflammatory syndrome that has been associated with systemic exposure to high doses of adeno-associated virus (AAV) gene therapy.

"Given the limited published research on HLH related to gene therapy, we believe it is important to provide information to the AAV gene therapy community on effective approaches to monitor, treat and reverse cases of HLH," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "In our ongoing clinical trial of NGN-401 gene therapy for Rett syndrome, we have implemented early monitoring and a treatment plan into our protocol, and we encourage others to do the same."

The 1E15 vg dose level Neurogene is moving forward in its Phase 1/2 trial of NGN-401 gene therapy for Rett syndrome translates into the E13 vg/kg range, and the Company is not aware of any case of HLH ever being reported at this dose level.

Oral Presentation Details

- **Title:** Hemophagocytic Lymphohistiocytosis (HLH)/Hyperinflammatory Syndrome Following High Dose AAV9 Therapy
- **Session:** Clinical Trial Spotlight Symposium
- **Date:** Friday, May 16
- **Abstract Presentation Time:** 3:00 p.m. CT
- **Location:** NOLA Theater C

Dr. McMinn will also participate in the following ASGCT sessions:

- **Title:** Fireside Chat: Reviving Hope in Deprioritized Cell and Gene Therapy Programs
- **Date:** Wednesday, May 14
- **Time:** 3:45 p.m. CT
- **Location:** Room 393-396

- **Title:** FDA's START Pilot Program in Action: Insights from Year One
- **Date:** Thursday, May 15
- **Time:** 3:45 p.m. CT
- **Location:** Room 393-396

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.

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 safety of NGN-401; the effectiveness of the monitoring protocol for HLH; and the ability to reverse cases of AAV-related HLH. 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, 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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