



## Neurogene Announces Upcoming Presentation of Safety Data from Phase 1/2 Trial of NGN-401 Gene Therapy for Rett Syndrome at ASGCT Meeting

April 22, 2024

*NGN-401 has been generally well-tolerated by three patients with multiple months of follow-up*

NEW YORK--(BUSINESS WIRE)--Apr. 22, 2024-- 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 that initial safety and tolerability data from its ongoing Phase 1/2 gene therapy clinical trial for Rett syndrome will be presented at the American Society for Gene and Cell Therapy (ASGCT) Annual Meeting. The data show that the NGN-401 gene therapy candidate has been generally well-tolerated, and there have been no treatment-emergent or procedure-related serious adverse events or signs of MeCP2 overexpression-related toxicity observed in any patient, including a patient with a mild variant predicted to result in residual MeCP2 function.

"We look forward to sharing the initial, favorable safety data of NGN-401, which is designed to use our differentiated EXACT transgene regulation technology and deliver a full-length *MECP2* gene to key areas of the brain underlying the pathophysiology of Rett syndrome," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "Rett syndrome is a complex neurological disorder with a narrow therapeutic window, and conventional gene therapy approaches have been unable to provide therapeutic protein levels without detrimental overexpression. Therefore, we believe it is important to share this safety update at the ASGCT Meeting, in advance of our expected fourth quarter 2024 interim efficacy read-out, as we have multiple months of data from three patients showing NGN-401 has been generally well-tolerated."

### Neurogene's ASGCT Meeting Poster Presentation Details

- **Title:** Preliminary Safety Results from the Ph1/2 study of NGN-401, a Novel Regulated Gene Therapy for Rett Syndrome
- **Date:** May 9, 2024
- **Time:** 12:00 p.m. ET
- **Location:** Exhibit Hall

The abstract can be accessed at this [link](#).

### 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](http://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 therapeutic potential and utility, efficacy and clinical benefits of NGN-401; the safety and tolerability profile of NGN-401; anticipated timing of interim clinical trial results from the Company's NGN-401 Phase 1/2 clinical trial for Rett syndrome; and expectations regarding the presentation of data at the upcoming ASGCT Annual Meeting. 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: risks related to the timing and success of enrolling patients in the expanded cohort of Neurogene's Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome; the expected timing and results of dosing of patients in the Company's clinical trials, including NGN-401; the potential that Neurogene may not be able to expand its Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome into the UK based on a variety of factors, including but not limited to any decisions of regulatory authorities, costs of expanding the trial in the UK, the availability of suitable clinical test sites, and the ability to enroll patients in the UK or other reasons; the potential for negative impacts to patients resulting from using a higher dose of NGN-401 in Cohort 2 of the Phase 1/2 clinical trial for the treatment of Rett syndrome; the risk that the Company may not be able to report its data on the predicted timeline; our limited operating history; the risk that Neurogene may not be able to raise adequate additional capital to finance its operations, complete its clinical trials and commercialize its products; risks related to Neurogene's ability to obtain regulatory approval for, and ultimately commercialize, its product candidates, including NGN-401; risks related to the outcome of non-clinical testing and early clinical trials for the Company's product candidates, including the ability of those trials to satisfy relevant governmental or regulatory requirements; risks related to Neurogene's limited experience in designing clinical trials and lack of experience in conducting clinical trials; and other risks and uncertainties identified under the heading "Risk Factors" included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023, filed with the Securities and Exchange Commission ("SEC") on March 18, 2024, 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.

This communication contains hyperlinks to information that is not deemed to be incorporated by reference into this communication.

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