Neurogene Announces RMAT Designation for NGN-401 Investigational Gene Therapy for Rett Syndrome

August 7, 2024

Designation based on preliminary clinical evidence from ongoing NGN-401 clinical trial that shows potential to address unmet medical needs in Rett syndrome

RMAT designation provides an opportunity for an Accelerated Approval pathway under the 21st Century Cures Act, and is in addition to NGN-401's selection by the FDA for the START Pilot Program

NEW YORK--(BUSINESS WIRE)--Aug. 7, 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 NGN-401 received Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA) for the treatment of Rett syndrome. The designation was based on preliminary clinical evidence from the ongoing Phase 1/2 clinical trial with NGN-401 that shows potential to address unmet medical needs in this disease.

"We appreciate the FDA's ongoing commitment to expedite development of our NGN-401 gene therapy for Rett syndrome, with RMAT designation following NGN-401's selection for the FDA's START Pilot Program, two synergistic initiatives designed to more rapidly advance promising treatments for patients with unmet medical needs," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "RMAT and START designations underscore the therapeutic potential of NGN-401 for Rett syndrome and reflect our commitment to accelerate development of NGN-401. We continue to expect to share interim efficacy data from the low-dose cohort in the fourth quarter of this year, with plans for additional data, including from the high-dose cohort, in the second half of 2025."

RMAT designation is granted for regenerative medicines intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition, with preliminary clinical evidence that indicates that the drug has the potential to address unmet medical needs. Benefits of the RMAT designation program include all the benefits of Fast Track and Breakthrough Therapy designation programs, including early and frequent communications with FDA senior managers, intensive guidance on efficient drug development and eligibility for an Accelerated Approval pathway and Priority Review.

In addition, NGN-401 was previously selected by the FDA for its Support for clinical Trials Advancing Rare disease Therapeutics (START) Pilot Program based on potential for clinical benefit and clinical development program readiness. The START Program is designed to provide the Sponsor access to more frequent and ad hoc interactions with FDA staff to facilitate program development and an expectation of generating high quality and reliable data to support a potential future marketing application.

About NGN-401

NGN-401 is an investigational AAV9 gene therapy being developed as a one-time treatment for Rett syndrome. It is the first clinical candidate to deliver the full-length human *MECP2* gene under the control of Neurogene's EXACT™ transgene regulation technology. EXACT technology is an important advancement in gene therapy for Rett syndrome, specifically because the disorder requires a treatment approach that enables targeted levels of *MECP2* transgene expression without causing overexpression-related toxic effects associated with conventional gene therapy.

NGN-401 was selected by the U.S. Food and Drug Administration (FDA) for its START Pilot Program. In addition to Regenerative Medicine Advance Therapy (RMAT) designation, NGN-401 previously received orphan drug designation, Fast Track designation and rare pediatric designation from the FDA. Neurogene was previously granted an INTERACT meeting with the FDA regarding the EXACT technology. NGN-401 also received orphan designation and advanced therapy medicinal product designation from the European Medicines Agency (EMA) and the Innovative Licensing and Application Pathway (ILAP) designation from the United Kingdom (UK) Medicines and Healthcare products Regulatory Agency (MHRA).

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 is expected to support pivotal clinical development activities. For more information, visit www.neurogene.com.

Cautionary Note Regarding Forward-Looking Statements

Statements in this press release that 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; trial designs, clinical development plans and timing of the presentation of clinical trial data for NGN-401, and the anticipated benefits of the FDA's RMAT designation as well as 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: risks related to the potential for negative impacts to patients dosed in the ongoing Phase 1/2 clinical trial for NGN-401, including patients in Cohort 2 receiving a high dose of NGN-401; the risk that the Company may not be able to report its data on the predicted timeline; risks related to Neurogene's ability to effectively use the RMAT designation or the START program to accelerate development of NGN-401 or its ability to obtain regulatory approval for, and ultimately commercialize, NGN-401 at all; 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, or its Quarterly Report on Form 10-Q for the quarter ended March 31, 2024, filed with the SEC on May 10, 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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