



## Neurogene Announces Closing of Merger with Neoleukin Therapeutics and Concurrent Private Placement of \$95 Million

December 19, 2023

*Neurogene focused on advancing Phase 1/2 trial for NGN-401, a differentiated clinical stage gene therapy to treat Rett syndrome using its EXACT technology; interim clinical data expected in 4Q24*

*Two patients successfully dosed with NGN-401, which has been well tolerated to date with no treatment-emergent or procedure-related serious adverse events, or transgene-related overexpression toxicity*

*Post transaction cash, cash equivalents, and investments of approximately \$200 million expected to fund advancement of Neurogene's EXACT gene therapy portfolio into 2H26*

*Shares to trade on NASDAQ under the new ticker "NGNE"*

NEW YORK--(BUSINESS WIRE)--Dec. 19, 2023-- Neurogene Inc. (NASDAQ: NGNE) ("Neurogene"), a clinical-stage company founded to bring life-changing genetic medicines to patients and families affected by rare neurological diseases, today announced the closing of its merger with Neoleukin Therapeutics, Inc. ("Neoleukin"). Neurogene shares are expected to begin trading on the NASDAQ Global Market under the ticker "NGNE" beginning today at the market open.

Concurrent with the closing of the merger, Neurogene closed an oversubscribed \$95 million private financing, led by new and existing healthcare-dedicated specialist and mutual fund institutional investors, including participation from Great Point Partners, EcoR1 Capital, Redmile Group, Samsara BioCapital, Janus Henderson Investors, funds and accounts managed by Blackrock, Casdin Capital, Avidity Partners, Arrowmark Partners, Cormorant Asset Management, Alexandria Venture Investments, and a healthcare investment fund. Neurogene's cash, cash equivalents, and investments of approximately \$200 million, before payment of final transaction-related expenses, are expected to fund operations and multiple potentially value-creating milestones into the second half of 2026.

"This transformative transaction provides us with a strong cash position allowing us to demonstrate the best-in-class potential of our EXACT transgene regulation technology in treating Rett syndrome, a debilitating and complex neurological disease that cannot be treated with conventional gene therapy," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "We look forward to expanding our ongoing Phase 1/2 clinical trial in 2024 for pediatric patients with Rett syndrome beyond the first cohort of five patients, and presenting interim clinical data from this study in the fourth quarter of 2024, with additional data from an expanded number of patients expected in the second half of 2025."

NGN-401 is an investigational adeno-associated virus (AAV9) gene therapy candidate for Rett syndrome purposefully designed and administered to maximize therapeutic activity while averting transgene overexpression toxicities. NGN-401 delivers the full-length human methyl cytosine binding protein 2 (*MECP2*) gene, providing an optimal gene replacement approach. NGN-401 leverages Neurogene's novel and proprietary EXACT transgene regulation technology, which provides a highly controlled and consistent MeCP2 expression on a cell by cell basis, and avoids overexpression-related toxicities associated with conventional gene therapy.

Neurogene [recently announced](#) the dosing of the first two patients with NGN-401 in the third and fourth quarters of 2023. Data from the ongoing Phase 1/2 clinical trial demonstrate that NGN-401 has been well tolerated, with no treatment-emergent serious adverse events or procedure-related events, and no signs of treatment-related overexpression toxicity. NGN-401 has been granted Orphan Drug Designation, Rare Pediatric Disease Designation, and Fast Track Designation by the U.S. Food and Drug Administration (FDA).

Neurogene is also developing NGN-101 for the treatment of CLN5 Batten disease, with interim clinical data for NGN-101 expected in the second half of 2024, and is advancing multiple discovery-stage candidates leveraging its EXACT transgene regulation technology. Neurogene expects to initiate a clinical study of one product candidate from its discovery-stage portfolio in 2025.

### Transaction Details

To ensure the combined company's compliance with the minimum bid price requirement of \$4.00 per share for initial listing on The Nasdaq Global Market, Neoleukin implemented a reverse split of its common stock at a ratio of 1-for-4 shares. In the reverse stock split, every four shares of Neoleukin common stock outstanding were combined and reclassified into one share of Neoleukin common stock. Immediately thereafter, and pursuant to the terms of the previously announced merger agreement, Neurogene

became a wholly owned subsidiary of Neoleukin upon completion of the merger, and each outstanding share of Neurogene common stock was converted into 0.0756 shares of Neoleukin common stock. Following the closing of the merger, there are approximately 16,887,060 shares of the combined company's common stock outstanding (assuming the exercise in full of all pre-funded warrants), with prior Neurogene stockholders, including investors in the private placement, owning approximately 84% and prior Neoleukin stockholders owning approximately 16% of the combined company's outstanding securities. The combined company will be led by Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene, and other members of the Neurogene management team.

TD Cowen served as exclusive financial advisor to Neurogene. TD Cowen and Stifel served as placement agents for Neurogene's concurrent private financing. Gibson Dunn & Crutcher LLP served as legal counsel to Neurogene and Cooley LLP served as legal counsel to the placement agents. Leerink Partners served as the exclusive financial advisor to Neoleukin. Fenwick & West LLP served as legal counsel to Neoleukin.

## **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 by 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**

This communication contains forward-looking statements (including within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, and Section 27A of the Securities Act of 1933, as amended (Securities Act)). 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. 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," 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. Statements that are not historical facts are forward-looking statements. Forward-looking statements in this communication include, but are not limited to, statements regarding the expected enrollment of and timing of data from Neurogene's Phase 1/2 clinical trial; statements regarding the potential of, and expectations regarding, Neurogene's programs, including NGN-101, NGN-401 and its research stage opportunities; the expected dosing of additional patients in Neurogene's Phase 1/2 clinical trial; statements by Neurogene's Founder and Chief Executive Officer; statements regarding the sufficiency of Neurogene's capital resources and cash runway. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: Neurogene's limited operating history; the significant net losses incurred since inception of Neurogene; the ability to raise additional capital to finance operations; the ability to advance product candidates through non-clinical and clinical development; the ability to obtain regulatory approval for, and ultimately commercialize, Neurogene's product candidates; the outcome of non-clinical testing and early clinical trials for Neurogene's product candidates, including the ability of those trials to satisfy relevant governmental or regulatory requirements; Neurogene's limited experience in designing clinical trials and lack of experience in conducting clinical trials; the ability to identify and pivot to other programs, product candidates, or indications that may be more profitable or successful than Neurogene's current product candidates; expectations regarding the market and potential for Neurogene's current product candidates; the substantial competition Neurogene faces in discovering, developing, or commercializing products; expectations regarding the potential tolerability, safety or efficacy for Neurogene's current product candidates; the ability to attract, hire, and retain skilled executive officers and employees; the ability of Neurogene to protect its intellectual property and proprietary technologies; reliance on third parties, contract manufacturers, and contract research organizations; and legislative, regulatory, political and economic developments and general market conditions. The foregoing review of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors included in Neoleukin's most recent Annual Report on Form 10-K, Quarterly Reports on Form 10-Q and Current Reports on Form 8-K filed with the Securities and Exchange Commission (SEC), the registration statement on Form S-4 filed with the SEC by Neoleukin and other documents to be filed by Neurogene from time to time with the SEC, discussions of potential risks, uncertainties and other important factors in Neurogene's subsequent filings with the SEC, as well as risk factors associated with companies, such as Neurogene, that operate in the biopharma industry. These forward-looking statements involve a number of risks, uncertainties (some of which are beyond Neurogene control) or other assumptions that may cause actual results or performance to be materially different from those expressed or implied by these forward-looking statements. 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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**Neurogene Contacts:**

Investor Relations:

Melissa Forst

Argot Partners

[Neurogene@argotpartners.com](mailto:Neurogene@argotpartners.com)

Media:

David Rosen

Argot Partners

[david.rosen@argotpartners.com](mailto:david.rosen@argotpartners.com)

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