Neurogene Reports First Quarter 2024 Financial Results and Highlights Recent Updates

May 10, 2024

Presented favorable safety data from Phase 1/2 NGN-401 gene therapy trial for Rett syndrome at ASGCT Annual Meeting

Received Australian HREC approval for NGN-401 trial

Remains on track to provide interim NGN-401 efficacy data from Cohort 1 in 4Q:24

Strong balance sheet with cash runway into 2H:26

NEW YORK--(BUSINESS WIRE)--May 10, 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 first quarter 2024 financial results and highlighted recent corporate updates.

"We have made substantial progress in our NGN-401 Rett syndrome gene therapy program since the beginning of the year, including dosing the third patient, expanding the trial to include additional patients and a high-dose cohort, and the recent clearance to conduct the trial in Australia," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "We were pleased to present data at the ASGCT Annual Meeting earlier this week, which continued to show that NGN-401 has been generally well-tolerated. We remain on track to release interim efficacy data from the low-dose cohort in the fourth quarter of 2024."

Continued Dr. McMinn, "The NGN-401 data support our strategy to expand into additional disease areas that could benefit from gene therapy with transgene regulation, and we continue to plan to advance an additional product candidate into the clinic in 2025. We remain in a strong financial position with cash runway into the second half of 2026."

First Quarter 2024 and Recent Highlights, and Anticipated Milestones

Phase 1/2 Trial of NGN-401 Gene Therapy for Treatment of Rett Syndrome

- Presented favorable safety data from the first three pediatric patients in low-dose Cohort 1 at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting:
  - NGN-401 has been generally well-tolerated in all three patients with approximately nine, six and three months of follow-up, respectively
  - All treatment-related adverse events (AEs) have been mild/Grade 1, and all laboratory value changes are known risks of AAV administration and asymptomatic
  - No signs or symptoms of MeCP2 overexpression toxicity reported in any patient, including Patient 1 who is nine months post-dosing and has a mild *MECP2* variant predicted to result in residual MeCP2 expression
  - No treatment-emergent or intracerebroventricular procedure-related serious AEs
- Announced today acknowledgment from the Australian Therapeutic Goods Administration and approval from the Human Research Ethics Committee (HREC) to conduct the Phase 1/2 clinical trial for NGN-401 in Australia, the third region in which the trial is cleared
- Continues to expect to report interim clinical data, including efficacy data from Cohort 1, in the fourth quarter of 2024; additional interim data, including from Cohort 2, are expected in the second half of 2025
- Previously expanded the trial to include a high-dose Cohort 2 and more patients in low-dose Cohort 1; these updates are expected to generate a more complete data package and inform the design of a future NGN-401 registrational study
- Remains on track to complete enrollment in Cohort 1 in the second half of 2024 and to begin enrollment in Cohort 2 in the second quarter of 2024

Phase 1/2 Trial of NGN-101 Gene Therapy for Treatment of CLN5 Batten Disease

- Continuing enrollment in high-dose Cohort 3, and plans to provide interim clinical data and a regulatory update in the second half of 2024; given the rarity of the disease, U.S. Food and Drug Administration alignment on a streamlined registrational pathway will be critical for continued investment in the program

Additional Corporate Updates

- Advancing early-stage portfolio, and anticipates an additional product candidate using transgene regulation technology to enter the clinic in 2025

Upcoming Events

- 5th Annual Goldman Sachs Global Healthcare Conference: Management will provide a corporate presentation on June 12 at 1:20 p.m. ET and participate in 1x1 meetings
- 2024 IRSF (International Rett Syndrome Foundation) Rett Syndrome Scientific Meeting: Presentation of safety data from the Phase 1/2 NGN-401 gene therapy trial for Rett syndrome on June 18-19, 2024

First Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and investments as of March 31, 2024 were $169.5 million. Cash outflows pertaining to the transaction with Neoleukin Therapeutics, including the offering costs associated with the pre-closing financing, were $9.6 million for the quarter ended March 31, 2024. The Company expects current cash, cash equivalents and marketable securities to fund operations into the second half of 2026.
- **Research & Development ("R&D") Expenses:** R&D expenses were $13.5 million for the three months ended March 31, 2024 compared to $10.3 million for the three months ended March 31, 2023. The increase in R&D expenses was primarily driven by an increase in NGN-401 clinical trial costs, increased preclinical costs related to our early discovery programs, and an increase in compensation and benefits expenses due to an increase in R&D headcount.
- **General & Administrative ("G&A") Expenses:** G&A expenses were $5.2 million for the three months ended March 31, 2024 compared to $2.8 million for the three months ended March 31, 2023. The increase in G&A expenses was primarily driven by an increase in compensation and benefits expenses due to an increase in G&A headcount, professional fees, insurance, information technology and other costs associated with becoming a public company.
- **Net Loss:** Net loss was $16.9 million for the three months ended March 31, 2024 compared to net loss of $12.3 million for the three months ended March 31, 2023.

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 therapeutic potential and utility, efficacy and clinical benefits of NGN-401 and NGN-101; the safety and tolerability profile of NGN-401; trial designs, clinical development plans and timing for each of NGN-401 and NGN-101, including anticipated timing of enrollment in and clinical trial results from the Company's NGN-401 Phase 1/2 trial for Rett syndrome or NGN-101 Phase 1/2 trial for CLN5 Batten Disease; initiation of new clinical sites for NGN-401 in Australia; expected interactions with the FDA regarding NGN-101; nomination of additional preclinical product candidates; and our expected cash resources and liquidity. 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 our Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome; the expected timing and results of dosing of patients in our clinical trials, including NGN-401 and NGN-101; the potential that we may not be able to expand our Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome into Australia based on a variety of factors, including but not limited to any decisions of regulatory authorities, costs of expanding the trial in Australia, the availability of suitable clinical test sites, and the ability to enroll patients in Australia, 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 we may not be able to report our data on the predicted timeline; risks related to our ability to obtain regulatory approval for, and ultimately commercialize, our product candidates, including NGN-401; and other risks and uncertainties identified under the heading "Risk Factors" included in our 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 our Quarterly Report on Form 10-Q for the quarter ended March 31, 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.

- Financial Tables Follow -

Neurogene Inc.
Condensed Consolidated Balance Sheets
(In Thousands of U.S. dollars)

	March 31, 2024	December 31, 2023
Assets		
Cash and cash equivalents	$ 150,140	$ 148,210
Other current assets	24,001	52,138
Non-current assets	21,209	22,225
Total assets	$ 195,350	$ 222,573
Liabilities		
Current liabilities	$ 11,818	$ 22,973
Non-current liabilities	12,755	13,576
Total liabilities	24,573	36,549
Stockholders' equity	170,777	186,024
Total liabilities and stockholders' equity	$ 195,350	$ 222,573

Neurogene Inc.
Condensed Consolidated Statements of Operations
(In thousands of U.S. dollars, except share information)

	Three Months Ended March 31,	
	2024	**2023**
Operating expenses:		
Research and development	$ 13,541	$ 10,283
General and administrative	5,238	2,752
Total operating expenses	18,779	13,035
Loss from operations	(18,779)	(13,035)
Other income, net	1,858	772
Net loss	$ (16,921)	$ (12,263)
Per share information: [1]		
Net loss per share, basic and diluted	$ (1.00)	$ (28.28)
Weighted-average shares of common stock outstanding, basic and diluted	16,903,735	433,623

[1] For the three months ended March 31, 2023, net loss per share information is presented for the Company's then outstanding Class A common stock. For the three months ended March 31, 2024, net loss per share information is presented for the Company's common stock. See Note 1, *Reverse Merger and Pre-Closing Financing* and Note 3, *Net Loss Per Share Attributable to Common Stockholders*, for additional information.

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