Neurogene Reports Second Quarter 2024 Financial Results and Highlights Recent Updates

August 9, 2024

NGN-401 gene therapy for Rett syndrome received RMAT designation from FDA based on preliminary clinical evidence indicating the potential to address unmet medical needs

NGN-401 selected for FDA START Program, also designed to accelerate development

Interim NGN-401 efficacy data from Cohort 1 remains on track for 4Q:24

NEW YORK--(BUSINESS WIRE)--Aug. 9, 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 second quarter 2024 financial results and highlighted recent corporate updates.

"We are pleased that NGN-401 gene therapy for Rett syndrome received RMAT designation, in addition to the recent selection by the U.S. FDA for its START Pilot Program, two programs that provide distinct opportunities for enhanced collaboration and communication with the FDA to accelerate NGN-401's development," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "We continued to advance the NGN-401 program with the first patient dosed in Cohort 2 in May, marking an important program milestone and demonstrating an early favorable safety profile with high-dose NGN-401, and reporting in mid-June that low-dose NGN-401 remained well-tolerated by the first three patients dosed in Cohort 1. As we look ahead, we continue to expect to release interim efficacy data from the low-dose cohort in the fourth quarter of this year, with plans to share additional low-dose as well as high-dose data in the second half of next year."

Second Quarter 2024 and Recent Highlights, and Anticipated Milestones

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

- NGN-401 received Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA) for the treatment of Rett syndrome, and was selected by the FDA for the Support for clinical Trials Advancing Rare disease Therapeutics (START) Pilot Program
  - RMAT designation requires preliminary clinical evidence to show the potential to address unmet medical needs for a serious or life-threatening disease or condition, and it provides opportunity for an Accelerated Approval pathway under the FDA's guidance
  - START selection criteria included potential for clinical benefit and clinical development and CMC program readiness, and the program provides opportunities for frequent advice and regular ad-hoc conversations with the FDA to address product-specific development topics
- Dosed the first patient in high-dose Cohort 2 in May, and reported in June that high-dose NGN-401 was well-tolerated with an early favorable safety profile
- Presented continued favorable safety profile data from the first three patients in low-dose Cohort 1 at the International Rett Syndrome Foundation (IRSF) ASCEND Summit in June, including:
  - No new treatment-related adverse events (AEs) since prior safety update in May 2024; all treatment-related AEs have been mild/Grade 1, and transient or resolving, and most AEs are known potential risks of AAV
  - No signs or symptoms indicative of MeCP2 overexpression toxicity reported, including in the patient with a mild genetic variant predicted to result in residual MeCP2 expression
  - No treatment-emergent or intracerebroventricular (ICV) procedure-related serious AEs
- 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
- Remains on track to complete enrollment in Cohort 1 in the second half of 2024

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

- Completed enrollment in the study, and now plans to provide interim clinical data and a regulatory update in the first quarter of 2025; given the rarity of CLN5 Batten disease, FDA alignment on a streamlined registrational pathway will be critical for continued investment in the program

Additional Corporate Updates

- Continues to expect an additional product candidate using transgene regulation technology will enter the clinic in 2025

Upcoming Events

- H.C. Wainwright 26th Annual Global Investment Conference: Management will provide a corporate presentation at 11:30 a.m. ET on September 9 and will participate in 1x1 meetings
- Cantor Global Healthcare Conference: Management will provide a corporate presentation at 2:30 p.m. ET on September 18 and participate in 1x1 meetings
- Cell & Gene Meeting on the Mesa: Management will participate in a “science slam” on neurological disease during the conference, which will be held October 7-9

Second Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and investments as of June 30, 2024 were $153.9 million. The Company continues to expect 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 $15.7 million for the three months ended June 30, 2024 compared to $10.3 million for the three months ended June 30, 2023. The increase in R&D expenses was primarily driven by an increase in NGN-401 clinical trial costs, increased preclinical costs related to the Company’s 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 $5.3 million for the three months ended June 30, 2024 compared to $2.3 million for the three months ended June 30, 2023. The increase in G&A expenses was primarily driven by an increase in employee-related expenses due to an increase in headcount, professional fees, rent, and other corporate-related expenses and market research costs.
- **Net Loss:** Net loss was $18.5 million for the three months ended June 30, 2024 compared to net loss of $11.9 million for the three months ended June 30, 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 and anticipated timing of clinical trial results for the Company’s NGN-101 Phase 1/2 trial for CLN5 Batten Disease; expected interactions with the FDA regarding NGN-101; expected benefits of RMAT designation and participation in the FDA’s START pilot program for NGN-401; 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 June 30, 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)

	June 30, 2024	December 31, 2023
Assets		
Cash and cash equivalents	$ 111,032	$ 148,210
Other current assets	48,117	52,138
Non-current assets	20,674	22,225
Total assets	$ 179,823	$ 222,573
Liabilities		
Current liabilities	$ 13,073	$ 22,973
Non-current liabilities	11,736	13,576
Total liabilities	24,809	36,549
Stockholders' equity	155,014	186,024
Total liabilities and stockholders' equity	$ 179,823	$ 222,573

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Revenue under licensing agreements [1]	$ 925	$ —	$ 925	$ —
Operating expenses:				
Research and development expenses	15,744	10,321	29,285	20,604
General and administrative expenses	5,315	2,275	10,553	5,027
Total operating expenses	21,059	12,596	39,838	25,631
Loss from operations	(20,134)	(12,596)	(38,913)	(25,631)
Other income, net	1,642	736	3,500	1,508
Net loss	$ (18,492)	$ (11,860)	$ (35,413)	$ (24,123)
Per share information: [2]				
Net loss per share, basic and diluted	$ (1.09)	$ (26.68)	$ (2.09)	$ (54.94)
Weighted-average shares of common stock	16,941,524	444,465	16,922,630	439,073

[1] The Company generated licensing revenue from the recognition of upfront payments received under the licensing and intellectual property assignment agreements with third parties to develop and commercialize legacy Neoleukin assets. Corresponding contingent value rights liabilities were recorded.

[2] For the three and six months ended June 30, 2023, net loss per share information is presented for the Company's then outstanding Class A common stock. For the three and six months ended June 30, 2024, net loss per share information is presented for the Company's common stock.

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