UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549
FORM 8-K

CURRENT REPORT
Pursuant to Section 13 OR 15(d) of The Securities Exchange Act of 1934

Date of Report (date of earliest event reported): May 12, 2026

Neurogene Inc.
(Exact name of registrant as specified in its charter)

Delaware	001-36327	98-0542593
(State or other jurisdiction of incorporation or organization)	(Commission File Number)	(I.R.S. Employer Identification No.)

535 W 24th Street, 5th Floor
New York, NY 10011
(Address of principal executive offices, including zip code)

Registrant's telephone number, including area code: (877) 237-5020

N/A
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.000001 par value	**NGNE**	**The Nasdaq Global Market**

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02 **Results of Operations and Financial Condition**

On May 12, 2026, Neurogene Inc. (the "Company") issued a press release announcing financial results for the quarter ended March 31, 2026. A copy of the press release announcing such results is attached as Exhibit 99.1 to this Current Report on Form 8-K. Also on May 12, 2026, the Company posted an updated corporate presentation on its website. A copy of the corporate presentation is furnished as Exhibit 99.2 to this Current Report on Form 8-K.

The information in this Item 2.02 and Exhibits 99.1 and 99.2 attached hereto are being furnished and shall not be deemed "filed" for the purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall such information or Exhibits 99.1 and 99.2 be deemed incorporated by reference into any filing under the Exchange Act or the Securities Act of 1933, as amended, except as expressly set forth by specific reference to such filing.

Item 9.01 **Financial Statements and Exhibits.**

(d) Exhibits

Exhibit Number	Description
99.1	Press Release dated May 12, 2026
99.2	Corporate Presentation (May 2026)
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

NEUROGENE INC.

Date: May 12, 2026

By: /s/ Christine Mikail
Name: Christine Mikail
Title: President, Chief Financial Officer

Neurogene Reports First Quarter 2026 Financial Results and Highlights Recent Updates

Dosed ~90% of participants in Embolden™ registrational trial of NGN-401 for Rett syndrome; on track to complete dosing in the second quarter of 2026

NGN-401 has been generally well-tolerated, with no cases of HLH at the 1E15 vg dose; additional interim Phase 1/2 data expected mid-2026

Presentation at ASGCT Meeting highlighted therapeutic rationale for ICV delivery in CNS-targeted gene therapy, including NGN-401

Strong cash position provides runway through first quarter of 2028

NEW YORK – May 12, 2026 – 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 2026 financial results and highlighted recent corporate updates.

"The significant unmet need in Rett syndrome and strong interest among physicians and caregivers continue to drive momentum for NGN-401, with approximately 90% of participants dosed in the Embolden™ registrational trial," stated Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "We continue to be encouraged that NGN-401 at the 1E15 vg dose has been generally well-tolerated. We are laser-focused on completing dosing in Embolden in the coming weeks and look forward to providing an update on this important clinical development milestone. In parallel, we are continuing to strengthen the organization and execute on early commercial-readiness activities to support a successful potential launch of NGN-401."

First Quarter 2026 and Recent Highlights

NGN-401 Gene Therapy for the Treatment of Rett Syndrome

- Announced today that ~90% of participants have been dosed in the Embolden registrational trial, and the Company is on track to complete dosing in the second quarter of 2026
- NGN-401 at the 1E15 vg dose has been generally well-tolerated, with no cases of hemophagocytic lymphohistiocytosis (HLH) in the Phase 1/2 trial or Embolden as of May 11, 2026
- Presented on the therapeutic rationale for intracerebroventricular (ICV) administration of central nervous system (CNS)-targeted gene therapy, including NGN-401, at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting

- Presentation highlighted the biological need to deliver gene therapy to the key regions of the brain underlying disease, supported by preclinical data demonstrating broader CNS biodistribution to brain regions central to Rett syndrome pathophysiology, as compared with intrathecal lumbar (IT-L) administration

Additional Corporate Updates

- Strengthened the leadership team with the appointment of Christy Shafer as Chief Commercial Officer and Christine Mikail, J.D., President and CFO of Neurogene, to the Board of Directors
  - Ms. Shafer brings more than 20 years of industry experience building and leading high-performing commercial organizations and launching therapies for rare neurological diseases
  - Ms. Mikail brings to the Board deep expertise in strategic transactions, capital formation, and corporate development, with a track record of building and positioning companies for long-term value creation

Key Anticipated NGN-401 Milestones in 2026

- Complete dosing of participants in the Embolden registrational trial in the second quarter of 2026
- Present updated interim safety and efficacy data on the pediatric and adolescent/adult cohorts from the Phase 1/2 trial, including at least 12 months of follow-up for all 10 participants, in mid-2026
- Initiate Process Performance Qualification (PPQ) campaign in mid-2026
- Continue early commercial-readiness activities

Upcoming Events

- H.C. Wainwright 4th Annual BioConnect Investor Conference: Management will participate in a fireside chat at 11:30 a.m. ET on May 19 and participate in investor meetings
- Goldman Sachs 47th Annual Global Healthcare Conference: Management will participate in a fireside chat at 2:40 p.m. ET on June 8 and participate in investor meetings

First Quarter 2026 Financial Results

- **Cash, Cash Equivalents and Short-Term Investments**: Cash, cash equivalents and short-term investments as of March 31, 2026 were $243.2 million and are expected to fund planned operations through the first quarter of 2028.
- **Research & Development (R&D) Expenses**: R&D expenses were $25.2 million for the three months ended March 31, 2026, compared to $17.8 million for the three months ended March 31, 2025. The increase in R&D expenses for the three months ended March 31, 2026 was primarily driven by an increase in Rett syndrome clinical trial costs of

NGN-401, chemistry, manufacturing and controls (CMC) costs to support NGN-401 and employee-related expenses due to an increase in R&D headcount. The increase was partially offset by decreases in spending on the CLN5 Batten disease program and early discovery.

- **General & Administrative (G&A) Expenses**: G&A expenses were $8.2 million for the three months ended March 31, 2026 and the three months ended March 31, 2025. Higher costs related to corporate and pre-commercial activities, along with increased employee-related expenses and professional fees, were offset by a decrease in non-cash stock-based compensation expense.
- **Net Loss**: Net loss was $30.9 million for the three months ended March 31, 2026, compared to $22.6 million for the three months ended March 31, 2025.

About Neurogene
Neurogene (NASDAQ: NGNE) is a clinical-stage biotechnology company focused on developing life-changing genetic medicines for people and their families impacted by devastating neurological diseases. The Company is using a biology-first approach paired with optimized delivery to develop purpose-built genetic medicines, including programs powered by its novel and proprietary EXACT™ transgene regulation technology. Neurogene is advancing its lead gene therapy program, NGN-401, as a potential best-in-class, one-time treatment for Rett syndrome. For more information, visit neurogene.com or follow on LinkedIn.

About NGN-401
NGN-401 is an investigational AAV9 gene therapy in late-stage clinical development as a potential best-in-class, one-time treatment for Rett syndrome. It is the only clinical candidate to deliver the full-length human *MECP2* gene and includes Neurogene's EXACT™ transgene regulation technology, which is designed to deliver consistent, tightly controlled MeCP2 protein expression on a cell-by-cell basis. NGN-401 is delivered through intracerebroventricular administration to achieve the broadest targeting directly to the brain and nervous system based on nonclinical biodistribution data. NGN-401 is being evaluated in the Embolden™ registrational clinical trial. Interim data from the Phase 1/2 trial (as of October 30, 2025) have shown that participants experienced multidomain, durable gains with continued skill acquisition observed over time, and NGN-401 at the 1E15 vg dose has been generally well-tolerated. NGN-401 has received Breakthrough Therapy, Regenerative Medicine Advanced Therapy, Fast Track, Orphan Drug and Rare Pediatric Disease designations and selection for the START Pilot Program from the U.S. Food and Drug Administration, Advanced Therapy Medicinal Product, Orphan and Priority Medicines designations from the European Medicines Agency and Innovative Licensing and Application Pathway designation from the United Kingdom Medicines and Healthcare products Regulatory Agency.

Cautionary Note Regarding Forward-Looking Statements
Statements in this press release are made as of the date of this press release. Neurogene does not undertake any obligation to make any updates to these statements to reflect events that occur or

circumstances that arise after the date of this press release, except as may be required under applicable U.S. securities law.

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; the applicability of reported interim results from the NGN-401 Phase 1/2 clinical trial to other participants or potential participants, including adolescent or adult patients; the potential for NGN-401 to be a best-in-class gene therapy for Rett syndrome; trial designs and clinical development plans for the Company's Embolden registrational clinical trial of NGN-401 for Rett syndrome, including timing of anticipated dosing and completion of participant dosing in the Company's clinical trial and the expected timeline of its PPQ activities for its CMC requirements; the response rate, expected durability and deepening of clinical data results from our NGN-401 clinical trial; the potential for future approval for commercialization of NGN-401 as a treatment for Rett syndrome; commercial launch readiness for NGN-401; expected timing for release of additional data from the Company's Phase 1/2 clinical trial of NGN-401; the potential for success of the Embolden registrational clinical trial of NGN-401 for Rett syndrome; the clinical benefit of delivering NGN-401 via ICV administration; expected future interactions with or positions of the FDA, including the timing and outcome of any such interactions and anticipated benefits of any regulatory designation for NGN-401, including the FDA's Breakthrough Therapy designation, Rare Pediatric Disease designation, RMAT designation and participation in the FDA's START program; and the time period over which existing cash resources may be sufficient to fund the Company's operations. 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: the potential for negative impacts to participants in the Phase 1/2 clinical trial of NGN-401 for the treatment of Rett syndrome; the risk that the Company may not be able to report data on the predicted timeline; risks related to the Company's ability to obtain regulatory approval for, and ultimately commercialize, its product candidates, including NGN-401; risks related to timing of completing enrollment in the Embolden trial of NGN-401 for Rett syndrome; and other risks and uncertainties identified under the heading "Risk Factors" included in Neurogene's Annual Report on Form 10-K for the year ended December 31,

2025, filed with the Securities and Exchange Commission ("SEC") on March 24, 2026, the Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, filed with the SEC on May 12, 2026, 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.
Unaudited Condensed Consolidated Balance Sheet Data
(In thousands of U.S. dollars)

	March 31, 2026	December 31, 2025
Assets		
Cash and cash equivalents	$ 124,161	$ 103,845
Short-term investments	119,021	165,168
Other current assets	3,565	2,757
Non-current assets	16,394	16,834
Total assets	$ 263,141	$ 288,604
Liabilities		
Current liabilities	$ 18,866	$ 16,411
Non-current liabilities	6,492	7,306
Total liabilities	25,358	23,717
Stockholders' equity	237,783	264,887
Total liabilities and stockholders' equity	$ 263,141	$ 288,604

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

	Three Months Ended March 31,	
	2026	2025
Operating expenses:		
Research and development expenses	$ 25,150	$ 17,760
General and administrative expenses	8,199	8,159
Total operating expenses	33,349	25,919
Loss from operations	(33,349)	(25,919)
Other income, net	2,415	3,272
Net loss	$ (30,934)	$ (22,647)
Per share information:		
Net loss per share, basic and diluted	$ (1.39)	$ (1.08)
Weighted-average shares of common stock outstanding, basic and diluted	22,305,734	20,996,287

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NEUROGENE
EVERY BREAKTHROUGH
BEGINS WITH BELIEF
Corporate Presentation
May 2026

Disclaimer

Forward Looking Statements

This communication contains 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 its programs, including its EXACT™ technology and NGN-401; the potential for commercial approval of NGN-401 and the speed with which any such approval might be obtained; market opportunities for Neurogene's product candidates, including the estimated prevalence of Rett syndrome and expected levels of demand for NGN-401; the safety and tolerability profile of NGN-401; the applicability of reported interim results from the NGN-401 Phase 1/2 clinical trial to other participants or potential participants, including adolescent or adult participants; any extrapolation of interim trial results on the likelihood of gaining approval of NGN-401 from the FDA or any other regulator; trial designs and clinical development plans for the Embolden™ registrational clinical trial of NGN-401 for Rett Syndrome, including timing of anticipated enrollment and completion of the enrollment in this clinical trial and the timing and the expected timeline of our Process Performance Qualification (PPQ) activities for our chemistry, manufacturing and controls (CMC) requirements; the response rate, expected durability and deepening of clinical data results from the NGN-401 clinical trials; expected timing for release of additional data from the Phase 1/2 clinical trial of NGN-401; the potential superiority of ICV administration and delivery of a full MECP2 gene as against other potential gene therapies; the potential for NGN-401 to be a best-in-class or first-in-class gene therapy for Rett syndrome; patient, caregiver and KOL sentiments relating to priorities on selecting potential gene therapy treatments; expectations related to payer reimbursement for NGN-401 if approved, including estimates related to potential reimbursement rates, the ease of obtaining reimbursement and the sentiments of payers with respect to value placed on certain outcomes and any impact of potentially implementing an outpatient regimen for NGN-401; the ability to and speed with which Neurogene could convert existing clinical trial sites to commercial sites if it is successful in obtaining regulatory approval for the commercialization of NGN-401; the potential for success of the Embolden registrational clinical trial of NGN-401 for Rett Syndrome; expected future interactions with or positions of the FDA or foreign regulatory authorities, including the timing and outcome of any such interaction and anticipated benefits of any regulatory designation for Neurogene's product candidates, including the FDA's Breakthrough Therapy designation and RMAT designation, the EMA's PRIME designation and participation in the FDA's START program with respect to NGN-401; the benefits of Neurogene's in-house manufacturing capabilities; anticipated early-stage discovery and expectations regarding the initiation of future clinical trials for programs in development; the timing and achievement of any catalyst for value creation for Neurogene; and Neurogene's cash runway, including the time period over which existing cash resources may be sufficient to fund the Company's operations. 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 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: uncertainties regarding interactions with and feedback received from FDA staff regarding the Embolden registrational trial including the risk that the FDA could change its requirements for the Embolden trial; the ability to raise additional capital to finance operations; the ability of Neurogene to report its data on the predicted timeline; the ability of Neurogene to effectively use the RMAT designation, Breakthrough Therapy designation, PRIME designation or START program to accelerate development of NGN-401; the potential for negative impacts to patients dosed in the ongoing clinical trials for NGN-401; 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; Neurogene's limited experience in designing and conducting clinical trials; the ability to identify and pivot to other programs, product candidates that may be more profitable or successful than Neurogene's current product candidates; expectations regarding the potential tolerability, safety or efficacy for NGN-401; the ability to attract, hire, and retain skilled executive officers and employees; reliance on third parties, contract manufacturers, and contract research organizations; the ability of Neurogene to protect its intellectual property and proprietary technologies; risks related to Neurogene's ability to correctly estimate its operating expenses, including its projected cash runway; and legislative, regulatory, political and economic developments and general market conditions.

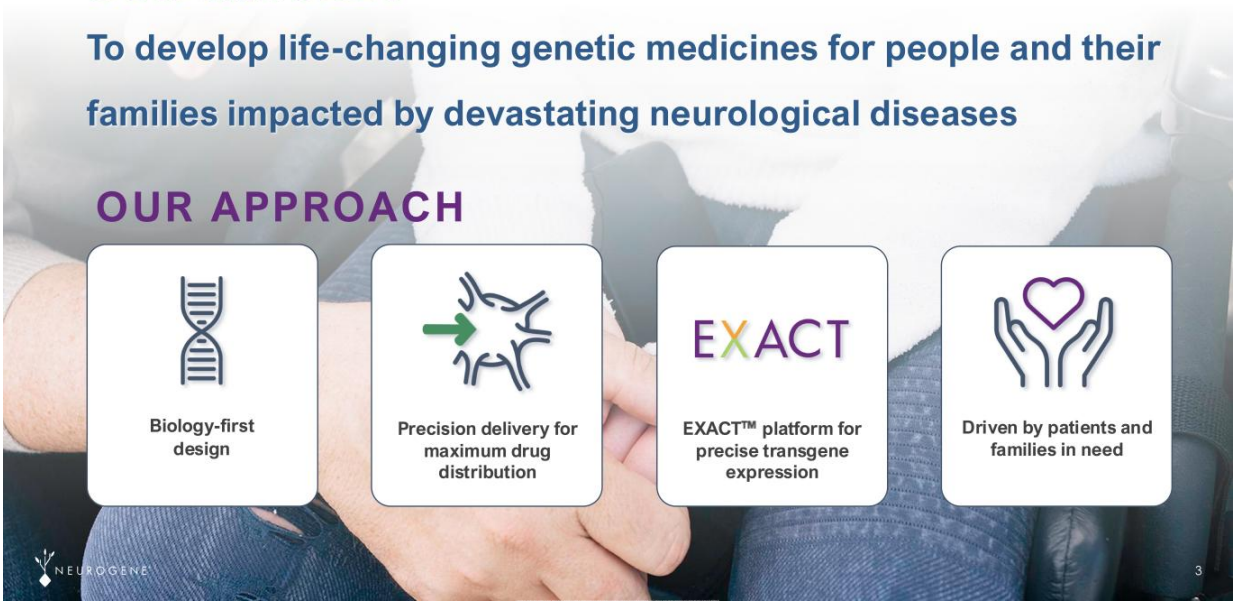
The foregoing review of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors included in the Company's most recent Annual Report on Form 10-K and Quarterly Reports on Form 10-Q filed with the Securities and Exchange Commission, 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's 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.

Industry and Market Data

Certain information contained in this Presentation relates to or is based on studies, publications, surveys and Neurogene's own internal estimates and research. In this Presentation, Neurogene relies on, and refers to, publicly available information and statistics regarding market participants in the sector in which Neurogene competes and other industry data. Any comparison of Neurogene to any other entity assumes the reliability of the information available to Neurogene. Neurogene obtained this information and statistics from third-party sources, including reports by market research firms and company filings. In addition, all of the market data included in this Presentation involve a number of assumptions and limitations, and there can be no guarantee as to the accuracy or reliability of such assumptions. Finally, while Neurogene believes its internal research is reliable, such research has not been verified by any independent source and Neurogene has not independently verified the information.

Trademarks

This Presentation may contain trademarks, service marks, trade names and copyrights of other companies, which are the property of their respective owners. Solely for convenience, some of the trademarks, service marks, trade names and copyrights referred to in this Presentation may be listed without the TM, SM © or ® symbols, but Neurogene will assert, to the fullest extent under applicable law, the rights of the applicable owners, if any, to these trademarks, service marks, trade names and copyrights.


OUR MISSION
To develop life-changing genetic medicines for people and their families impacted by devastating neurological diseases
OUR APPROACH
Biology-first design
Precision delivery for maximum drug distribution
EXACT
EXACT™ platform for precise transgene expression
Driven by patients and families in need
NEUROGENE
3

NGN-401 is Rapidly Advancing Towards Commercialization as a Potential Transformative One-Time Treatment for Rett Syndrome

Potential Best-in-Class Treatment for Rett Syndrome

- Compelling clinical evidence showing durable multidomain improvements demonstrated across full spectrum of disease severity
- Received FDA Breakthrough Therapy designation, in addition to START Pilot Program, RMAT and other regulatory designations

Clear Path to Registration with Single Registrational Trial for Broadest Age Range

- Key elements of Embolden™ registrational trial aligned with FDA, including a single trial for ages ≥ 3 years
- Dosed ~90% of participants in Embolden registrational trial

High-Value Rare Disease with Significant Opportunity to Improve Patient Lives

Early commercial-readiness activities underway to transform multi-billion-dollar market burdened by lifelong, high-intensity medical care

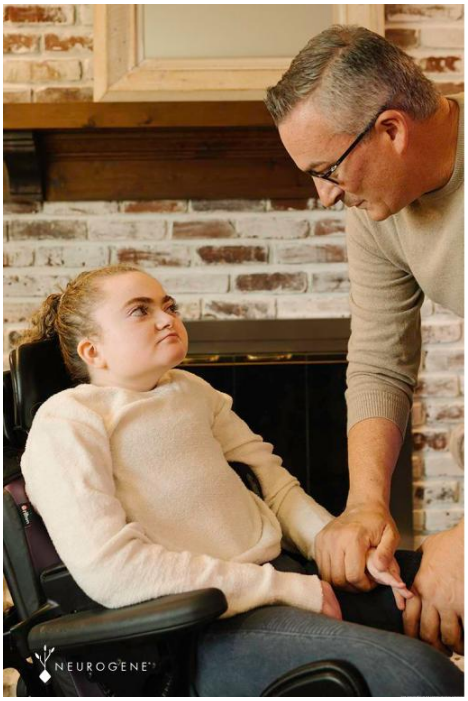
Upcoming Milestones

- Complete dosing of Embolden **on track for 2Q'26**
- Presentation of 12+ months Phase 1/2 data for all 10 participants **planned for mid-2026**
- Process Performance Qualification (PPQ) campaign **planned to begin mid-2026**

Neurogene is Positioned for Significant Value Inflection with Multiple Catalysts in 2026

NEUROGENE

NGN-401 for Rett Syndrome



Rett Syndrome: Rare, Debilitating, Progressive, Neurodevelopmental Disorder

Cause: Variants in the *MECP2* gene on the X chromosome lead to deficiency of functional MeCP2 protein

- MeCP2 is a DNA-binding protein essential for normal brain and nervous system function

Onset: Developmental delay occurs at 6-18 months, followed by loss of previously acquired milestones during regression and subsequent developmental plateau at ~3 years

Hallmark features:

- Loss of expressive and receptive communication
- Loss of purposeful hand function with repetitive movements
- Gait abnormalities and mobility challenges
- Seizures, breathing irregularities, severe constipation

Multi-Billion-Dollar Market Opportunity for Disease-Modifying Gene Therapy for Rett Syndrome

Rett Syndrome is One of the More Prevalent Rare Diseases

~15,000 – 20,000 patients
Major market prevalence
US, EU and UK[1]

1:10,000 females
Worldwide incidence[2]

Clear Unmet Need

No disease-modifying treatment available

Only treatment options are limited to symptom management

High burden on families and healthcare system
Lifelong, constant care is required

Payors are Receptive to Reimbursement[3]

Payors value functional changes that are clinically meaningful and show improvements in activities of daily living

Payors are familiar with Rett syndrome
Decades-long survival supports premium-priced durable gene therapy

[1] Major market prevalence based on internal estimates; U.S. prevalence estimate based on published incidence rates; Laurvick CL, et al. J Pediatr 2006;148(3):347–35.
[2] WW incidence estimate based on published incidence rates; Pini G, et al. Orphanet Journal of Rare Diseases (2016) 11:132.
[3] Internal market research

NGN-401: Purposefully Designed to Be the Best-in-Class Gene Therapy for Rett Syndrome

NGN-401 Construct

Full-length *MECP2* to translate functional MeCP2 protein

EXACT transgene regulation technology designed to safely achieve target levels

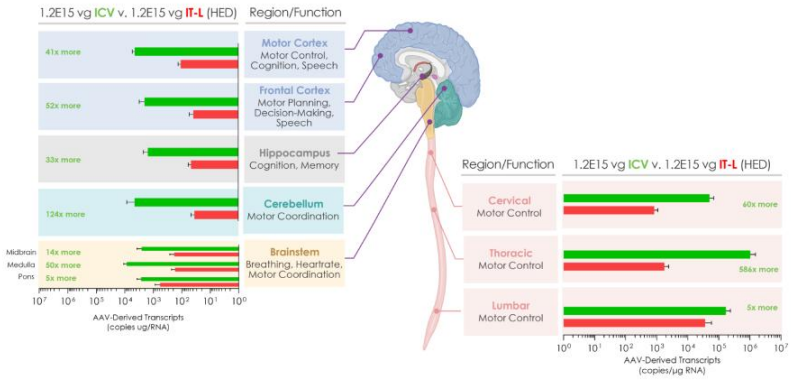
ICV delivery to achieve the broadest targeting of brain and nervous system

Only Gene Therapy Designed to Safely Deliver Full-length Protein to Key Areas of the Brain and Nervous System to Maximize Benefit

ICV Delivery Achieves Greater Expression in the Brain Compared to IT-L

ICV route of delivery has been shown in preclinical models to have greater targeting of brain and nervous system regions underlying Rett syndrome pathophysiology compared to IT-L

An estimated 30,000 ICV procedures are performed by neurosurgeons annually in the U.S. and require minimal downtime/recovery



ICV = Intracerebroventricular
IT-L = intrathecal lumbar
ESGCT 2025

NEUROGENE

NGN-401 PHASE 1/2 TRIAL

Dosing Completed in NGN-401 Phase 1/2 Clinical Trial in Females with Rett Syndrome

Trial evaluating 1E15 vg dose of NGN-401

n=8	n=2
Ages 4-10	*Ages ≥ 11*

Trial Overview

Open-label, multicenter trial designed to assess the safety, tolerability and efficacy of NGN-401

Key Eligibility Criteria:

- Females with Classic Rett syndrome in post-regression stage of illness
- Clinical diagnosis and genetic confirmation of pathogenic *MECP2* variant
- Clinical Global Impression-Severity (CGI-S) score of 4-6

Baseline Characteristics of the Pediatric Participants in Phase 1/2

Pediatric Cohort (n=8)								
	Pt:1	Pt:2	Pt:3	Pt:4	Pt:5	Pt:6	Pt:7	Pt:8
Age at Dosing (Years)	7	4	6	7	6	4	6	8
Baseline CGI-S Score	4 Moderately Ill	5 Markedly Ill	5 Markedly Ill	5 Markedly Ill	5 Markedly Ill	5 Markedly Ill	4 Moderately Ill	4 Moderately Ill
Genetic Variant Severity	Mild	Severe	Severe	Severe	Severe	Moderate	Mild-Moderate	Mild-Moderate
Time Post-Dosing (Months)	24	18	18	15	12	6	6	6

Evaluating the Impact of NGN-401 Across Full Spectrum of Disease Severity

NGN-401 Drove Clinically Meaningful and Durable Improvement Across Key Rett Syndrome Domains

Key Findings from Interim Phase 1/2 Data

- **100%** showed functional improvements across core disease domains – fine motor/hand function, gross motor/ambulation and communication
- **35** total developmental milestones gained
  - **No plateau**, including out to 24 months
  - **Multidomain gains** enable increasingly complex activities, enhancing independence and health-related quality of life
- **88%** achieved improved CGI-I score
- 1E15 vg dose continues to be **generally well-tolerated**, consistent with AAV-based gene therapy

Caregivers Seek Treatment that Improves Daily Living and Leads to Durable, Multidomain Improvements

Caregivers Consistently Highlight Improvements in Core Domains are the Priority

Fine Motor/Hand Function Improvements

- Enables self-feeding, quicker meals, family/social dining and independence
- E.g., Transferring/grasping objects, finger feeding, drinking from a cup, using utensils

Expressive & Receptive Communication Improvements

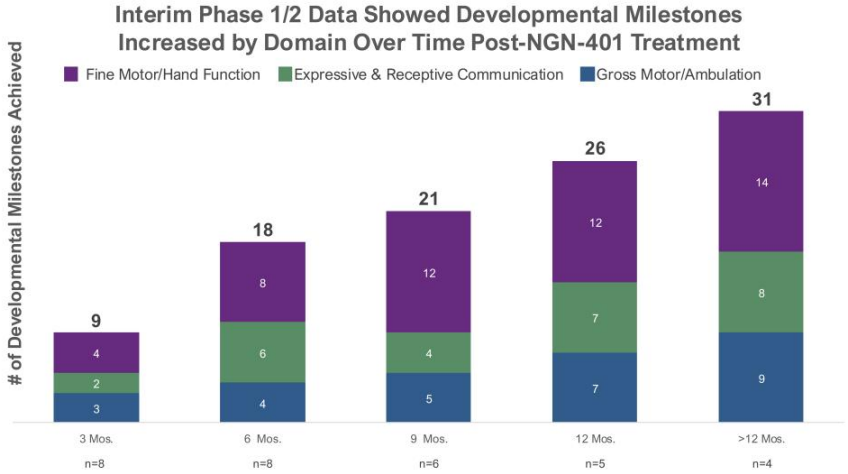
- Expressing needs, following instructions simplifying daily routines and strengthening connections
- E.g., Making choices, using words with meaning

Gross Motor/Ambulation Improvements

- Enhances mobility and reduce caregiver burden
- E.g., Sitting w/support, standing while holding on, climbing up/down stairs

Quantitative research conducted in 2024 with 30 Rett syndrome caregivers to daughters aged 3-22 years old;
Follow-up qualitative research conducted in 2024 and 2025 with 27 Rett syndrome caregivers (sub-set of quantitative respondents) to daughters aged 3-22 years old

NGN-401 Drove Durable Accumulation of Multidomain Milestones Across Core Domains That Matter Most to Caregivers



NGN-401 Delivers Durable, Multidomain Improvements that Translate to Real Life Impact

Improvements in Key Domains Lead to:

Multidomain improvements

Participation in activities of daily living

Improvements in health-related quality of life

New Functional Abilities and Improvements Post-Treatment with NGN-401

Fine Motor/Hand Function

Less dependence on caregivers for basic needs

Uses hands to self-feed, drink, play with toys, self-soothe

Decreased to no stereotypies

Uses utensils to self-feed

Gross Motor/Ambulation

Reduced physical burden for caregivers for activities of daily living

Sits without support

Walks up and down stairs unassisted

Bends at waist and stands back up

Bears more weight for ease of transfer

Can get on/off furniture, out of bathtub, into car unassisted

Communication

Can communicate needs & wants, and foster greater social connections

Says words with meaning

Follows daily routines, e.g., getting ready for school

Communicates needs and wants; can ID colors

Engages with friends and family; shows affection

Starting/ improved communication with use of assisted device

Autonomic

Enhanced comfort and reduced distress

Improvements in constipation

Drinks clear liquids, eats solids for first time

Absent aspiration pneumonia events

Sleeps through the night

Phase 1/2 Caregiver Testimonials Following Treatment with NGN-401 Highlight Improvements Important to Families

"**Sleep improved significantly** as she is **able to sleep all through the night without** any **issue**."

"Her **understanding has improved**, especially with me **when I ask her** to do small tasks, then **she will do it immediately almost every time**…"

"The other day she...**ran a couple steps** while out…"

"**She uses both hands to grab her blanket**...She **holds** a **banana and takes bites**."

"She is **paying attention**, and even at school with decision-making…**she knows what we are asking of her**."

"She reacts to…[the] puppy. We have never seen that…nothing going on before, nothing was worth her attention. **Now she is enjoying day-to-day**."

"She is **happier**."

"**I am grateful** [NGN-401] has seemed to **benefit [her] health and life**…"

"**She is so happy all the time and calm.**"

"Before, she was really stuck a lot. Her **gait has improved a lot**."

"**Walking and standing are so much better**. Before she would fall all the time and now she doesn't. **She is so much stronger and rarely falls**."

"She **holds onto things longer**…she is **eating pieces of fruit using a fork, a new skill**."

"She **kissed me** for the **first time ever**."

NGN-401 Remains Generally Well Tolerated at the1E15 vg Dose Level

	1E15 vg Dose Total n=10	
	N	Events
TEAEs related to NGN-401	9	59
Serious TEAEs Unrelated to NGN-401	3	4
Serious TEAEs Related to NGN-401	1	2

- All TEAEs related to NGN-401 have been Grade 1 (mild) or Grade 2 (moderate) in severity; the majority are known potential risks of AAV and have resolved or are resolving
  - Most participants experienced mild liver enzyme elevations
  - SAEs (Grade 2) related to abnormal nerve conduction findings occurred in Pt:5:
    - Areflexia and related elective inpatient diagnostic testing
    - Nerve conduction findings have returned to normal range
    - Unrelated to NGN-401, Pt:5 also experienced a leg fracture confounding her Month 12 gross motor assessment
- No evidence of hemophagocytic lymphohistiocytosis (HLH) in any participant
- Seizures have remained well controlled following NGN-401
- No intracerebroventricular (ICV) procedure-related AEs
- No signs or symptoms indicative of MeCP2 overexpression

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As of data cutoff date of October 30, 2025
TEAE = Treatment emergent adverse event

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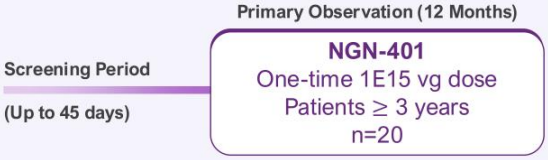
Embolden
A Clinical Study for Rett Syndrome

EMBOLDEN™
REGISTRATIONAL TRIAL OVERVIEW

Alignment with FDA on Single Registrational Trial to Support BLA Submission; Enrollment Underway in Embolden Trial

Embolden
A Clinical Study for Rett Syndrome

Single Arm, Baseline-Controlled, Open-Label Trial of NGN-401 in Females with Rett Syndrome



Primary Endpoint at 12 Months

Responder-based composite endpoint defined as:
- **CGI-I of ≤ 3** and
- **Gain from baseline of any one developmental milestone**

35% response rate, or 7 of 20 participants, needed for success

Key Secondary Endpoints

- **CGI-I score of ≤ 2**
- **Gain from baseline of at least 2 developmental milestones**

Developmental Milestones

- **Pre-specified from a list of 28**
- **Captured through standardized video recordings and rated by independent, central, blinded raters**

NEUROGENE® Immunosuppression: Steroid prophylaxis during first 90 days, followed by taper

28 Pre-Specified Developmental Milestones From Natural History Database Aligned with FDA, KOLs and Caregivers as Meaningful

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Fine Motor/ Hand Function

- Reached for toy
- Taken a drink from a cup held without assistance
- Used raking grasp to retrieve an object
- Used a pincer grasp (either refined or modified)
- Finger fed
- Transferred an object from one hand to the other
- Used a spoon/fork to eat without assistance

Gross Motor/Ambulation

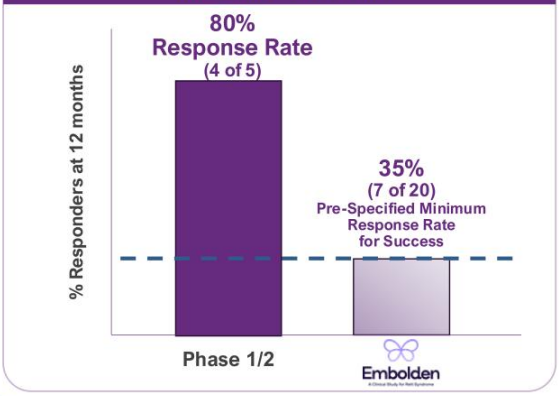
- Sat with support when placed
- Sat without support when placed
- Come to sitting
- Pulled to standing
- Stood while holding on
- Stood independently
- Cruised around furniture or holding on to someone
- Walked independently
- Climbed up stairs with help
- Climbed up stairs without help
- Climbed down stairs with help
- Climbed down stairs without help
- Ran 10 feet without falling

Communication

- Responded to familiar names/words
- Followed a command with a gesture
- Followed a command without a gesture
- Pointed for something they want
- Waved bye-bye
- Babbled
- Used words with meaning
- Spoken in phrases (2 words or more with meaning)

Interim Phase 1/2 Data and Key Trial Design Elements Carried Into the Registrational Trial De-risk Embolden Outcomes

Responders in Phase 1/2 Trial Exceed the Bar for Success Established as the Primary Endpoint in the Embolden Trial



Key Design Elements Carried Into Embolden

Phase 1/2	Embolden
1E15 vg dose	
Steroid-only immunosuppression regimen	
Trial sites at Rett Centers of Excellence	
Standardized CGI-I training at all sites	
Independent, central review of video-based milestone assessments based on pre-specified definitions	Independent, central, blinded review of video-based milestone assessments based on pre-specified definitions

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As of data cutoff date of October 30, 2025

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COMMERCIAL READINESS & KEY ANTICIPATED MILESTONES

Building a Strong Foundation for Commercialization

Positioning Embolden clinical trial sites for rapid conversion to commercial sites at launch

Embolden Clinical Trial Sites at Rett Centers of Excellence

Internal CMC capabilities and manufacturing facility to produce commercial product

Payor research confirms strong reimbursement potential for NGN-401

Separate payment to be issued for NGN-401, enabling hospitals to secure reimbursement without inpatient bundling constraints

Outpatient pathway optionality intended to further simplify reimbursement

Wholly Owned and Fully Integrated In-House AAV Manufacturing

- Flexibility to manufacture AAV product at low cost
- Own product quality and development timelines
- Process development expertise supports both HEK293 and Sf9/rBV manufacturing platforms
- Flexibility to rapidly adapt CMC execution to program needs

Process and scale are consistent between clinical and commercial; avoids potential future comparability challenges

NGN-401 is Positioned for Leadership in Rett Syndrome with Multiple Value-Creating Catalysts Expected in 2026 and Beyond

2025

- **Compelling Interim Clinical Data:** Multidomain, durable improvements across spectrum of disease severity
- **Single Registrational Trial Enrolling:** Enables approval for broad population
- **Early Commercial-Readiness Underway:** Robust payor and market research to support future product launch

ANTICIPATED 2026 MILESTONES

- **Complete Dosing in Embolden Registrational Trial**
  Expected Timing: 2Q'26
- **Present Phase 1/2 Data:** 12+ month data for all 10 participants
  Expected Timing: Mid-2026
- **Start PPQ campaign**
  Expected Timing: Mid-2026
- **Initiation of Additional Commercial-Readiness Activities**

Strong Cash Balance (1Q'28)

Expected to fund operations through Embolden data readout, BLA submission and key pre-launch activities

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APPENDIX:
INTERIM PHASE 1/2 DATA UPDATE
PARTICIPANT VIGNETTES

Pt:1 Gained 11 Developmental Milestones Across All Core Domains with Durability out to 24 Months

Pt:1 Baseline: 7 Yrs Old

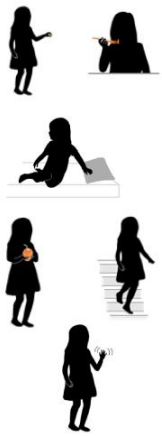
Raking, no ability to hold objects

Walking, ataxic gait, no ability to climb stairs

Severe impairment, unable to follow commands, indicate wishes

Pt:1 Developmental Milestones Post-NGN-401	Months Post-NGN-401						
	3	6	9	12	15	18	24
Fine Motor/Hand Function							
Used a pincer grasp (either refined or modified)		✓	✓	✓	✓	✓	✓
Taken a drink from a cup held without assistance		✓	✓	✓	✓	✓	✓
Used a spoon/fork to eat without assistance					✓	✓	✓
Transferred an object from one hand to the other					✓	✓	✓
Gross Motor/Ambulation							
Climbed up stairs without help		✓	✓	✓	✓	✓	✓
Heel-to-toe walking*			✓	✓	✓	✓	✓
Climbed down stairs without help				✓	✓	✓	✓
Come to sitting							✓
Communication							
Followed a command without a gesture		✓	✓	✓	✓	✓	✓
Waves bye bye				✓	✓	✓	✓
Pointed for something they want				✓	✓	✓	✓

Post Treatment with NGN-401



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As of data cutoff date of October 30, 2025

*Not included in Embolden developmental milestones list; previously reported developmental milestones from other validated scales

Images are representative of developmental milestones

Pt:2 Gained 10 Developmental Milestones Across All Core Domains with Durability out to 18 Months

Pt:2 Baseline: 4 Yrs Old

Pt:2 Developmental Milestones Post-NGN-401	Months Post-NGN-401					
	3	6	9	12	15	18
Severe impairment, unable to use hands — Fine Motor/Hand Function						
Reached for toy	✓	✓	✓	✓	✓	✓
Used raking grasp to retrieve an object			✓	✓	✓	✓
Finger fed			✓	✓	✓	✓
Taken a drink from a cup held without assistance			✓	✓	✓	✓
Impaired, ataxic, help to stand — Gross Motor/Ambulation						
Pulled to standing	✓	✓	✓	✓	✓	✓
Bent down, touched floor, and recovered*			✓	✓	✓	✓
Stepped off curb with help*				✓	✓	✓
Climbed up stairs with help					✓	✓
Severe impairment, unable to follow commands, non-verbal — Communication						
Followed a command without a gesture	✓	✓	✓	✓	✓	✓
Used words with meaning	✓	✓	✓	✓	✓	✓

Post Treatment with NGN-401

As of data cutoff date of October 30, 2025
*Not included in Embolden developmental milestones list; previously reported developmental milestones from other validated scales
Images are representative of developmental milestones

Pt:3 Gained 6 Developmental Milestones Across All Core Domains with Durability out to 18 Months

Pt:3 Baseline: 6 Yrs Old

Severe Dysphagia

Raking grasp

Cannot sit, stand or walk independently

Cannot follow commands

Pt:3 Developmental Milestones Post-NGN-401	Months Post-NGN-401					
	3	6	9	12	15	18
Fine Motor/Hand Function						
Used a pincer grasp (either refined or modified)		✓	✓	✓	✓	✓
Finger fed			✓	✓	✓	✓
Transferred an object from one hand to the other			✓	✓	✓	✓
Gross Motor/ Ambulation						
Sat without support when placed	✓	✓	✓	✓	✓	✓
Communication						
Followed command without gesture				✓	✓	✓
Followed command with gesture						✓

Post Treatment with NGN-401

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As of data cutoff date of October 30, 2025
Images are representative of developmental milestones

Pt:4 Gained 4 Developmental Milestones Across 2 Core Domains with Durability out to 15 Months

Pt:4 Baseline: 7 Yrs Old

Raking grasp, unable to hold objects

Unable to follow commands

Pt:4 Developmental Milestones Post-NGN-401	Months Post-NGN-401				
	3	6	9	12	15
Fine Motor/Hand Function					
Used a pincer grasp (either refined or modified)	✓	✓	✓	✓	✓
Used a spoon/fork to eat without assistance	✓	✓	✓	✓	✓
Taken a drink from a cup held without assistance	✓	✓	✓	✓	✓
Communication					
Followed command without a gesture		✓	✓	✓	✓

Post Treatment with NGN-401

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As of data cutoff date of October 30, 2025
Images are representative of developmental milestones

Pt:7 Gained 1 Developmental Milestone at Early Timepoint

Pt:7 Baseline: 6 Yrs Old

Could not use utensils

Pt:7 Developmental Milestones Post-NGN-401	Months Post-NGN-401	
	3	6
Fine Motor/Hand Function		
Used a spoon/fork to eat without assistance		✓

Post Treatment with NGN-401

As of data cutoff date of October 30, 2025
Images are representative of developmental milestones

Pt:8 Gained 3 Developmental Milestones Across 2 Core Domains at Early Timepoint

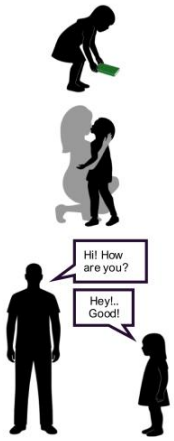
Pt:8 Baseline: 8 Yrs Old

Unable to bend at waist

Unable to follow commands, cannot use words with meaning

Pt:8 Developmental Milestones Post-NGN-401	Months Post-NGN-401	
	3	6
Gross Motor/ Ambulation		
Bent down, touched floor, and recovered*	✓	✓
Communication		
Followed command with a gesture		✓
Used words with meaning		✓

Post Treatment with NGN-401



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*Not included in Embolden developmental milestones list; previously reported developmental milestones from other validated scales
Images are representative of developmental milestones