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): June 29, 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 7.01 Regulation FD Disclosure

On June 29, 2026, Neurogene Inc. (the "Company") issued a press release announcing positive, updated clinical data from its Phase 1/2 trial evaluating NGN-401 gene therapy for the treatment of females with Rett syndrome. The Company will host a conference call and webcast today, Monday, June 29, 2026 at 8:00 a.m. Eastern Time, to discuss the data results A copy of the press release announcing such results is attached as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

The information in this Item 7.01 and Exhibit 99.1 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 Exhibit 99.1 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 8.01 Other Events

On June 29, 2026, the Company also made publicly available a presentation announcing positive, updated clinical data from its Phase 1/2 trial evaluating NGN-401gene therapy for the treatment of females with Rett syndrome. A copy of the presentation is filed as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Description
99.1	Press Release dated June 29, 2026
99.2	Corporate Presentation dated June 29, 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: June 29, 2026

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

Neurogene Reports Positive Long-term Clinical Data from Phase 1/2 Trial of NGN-401 Gene Therapy for Rett Syndrome

47 total developmental milestones gained across 10 participants, with durable treatment effect and no plateau or milestone loss through 30 months of follow-up

100% of participants improved in CGI-I score and gained ≥1 developmental milestone, with an average of 4.7 milestones per participant, and all gaining ≥1 developmental milestone in the past 12 months

Developmental milestones were gained in a progressive, stepwise sequence, suggesting a restart of development post-treatment

NGN-401 at the 1E15 vg dose remains generally well-tolerated (N=35) as of data cutoff date of June 16, 2026

Dosing complete in the Embolden™ registrational trial with no treatment-related SAEs or DLTs as of data cutoff date of June 16, 2026, with topline data anticipated in 2H 2027

Company to host investor/analyst webcast today at 8:00 a.m. ET

NEW YORK – June 29, 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 updated, positive data from its Phase 1/2 clinical trial evaluating NGN-401 gene therapy for the treatment of females with Rett syndrome.

Across 10 participants, 47 total developmental milestones were gained (average of 4.7 per participant), with both pediatric and adolescent/adult participants demonstrating clinical response and continued, progressive improvements through 30 months of follow-up, with no plateau or loss of milestones observed.
“We are encouraged by the long-term Phase 1/2 data across a broad age range and wide spectrum of disease severity, which we believe demonstrate that NGN-401 is driving gain of durable and clinically meaningful developmental milestones, and continued improvements were observed through 30 months of follow-up,” said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. “Importantly, the gains of developmental milestones are not isolated events – they build over time with participants acquiring multiple milestones across the core Rett syndrome functional domains of hand function, gross motor function and communication. This multidomain progression is not observed in the natural history of Rett syndrome, and we believe supports NGN-401’s differentiated profile, driven by its purposeful design and targeted delivery.”

“In typical development, children gain milestones in an organized, cumulative sequence, with each milestone building on the last; in Rett syndrome, that process is stopped by regression,” said Bernhard Suter, M.D., Medical Director of the Blue Bird Circle Rett Center at Texas Children’s Hospital, Associate Professor of Pediatrics and Neurology at Baylor College of Medicine, and principal investigator in the NGN-401 Phase 1/2 clinical trial. “These results show that the participants treated with NGN-401 are now gaining developmental milestones in a manner that suggests restarting developmental progression, something not observed in the natural history of the disease. As a result, participants are gaining greater independence and are more engaged in day-to-day activities with their families.”

Phase 1/2 Clinical Data as of Data Cutoff Date of June 16, 2026 (N=10, follow-up 12-30 months post-treatment)

Efficacy Data

- 100% of participants improved on the Clinical Global Impression-Improvement (CGI-I) scale and gained ≥1 developmental milestone, consistent with the composite endpoint used to evaluate efficacy in the Embolden™ registrational trial
- 47 total developmental milestones were gained across participants, averaging 4.7 milestones per participant
- All participants gained ≥1 developmental milestone in the past 12 months
- Participants gained milestones in a progressive, developmentally ordered stepwise sequence, suggesting a restart of developmental progression
- Rapid onset of treatment effect, with median time to first clinical improvement of 2 months post-treatment
- Milestone gains deepened over time, increasing by 95% from 6 to 12 months and 147% from 6 to ≥12 months
- 7 of 10 participants gained ≥2 developmental milestones and demonstrated improvements across ≥2 core Rett syndrome domains; both of these improvements were observed in pediatric and adolescent/adult participants
- Continued improvement through 30 months post-dose, with no plateau observed and no milestones lost
- Participants experienced clinically meaningful improvements across additional validated Rett syndrome scales, including the Rett Syndrome Gross Motor Scale (RSGMS) and Rett Syndrome Hand Function Scale (RSHFS) ($p<0.001$)

Safety Data

- NGN-401 at the 1E15 vg dose remains generally well-tolerated
- All treatment-related adverse events have been mild (Grade 1) or moderate (Grade 2) in severity, and the majority are known potential risks of AAV and have resolved or are resolving

- No new treatment-related SAEs reported since last data cutoff date in October 2025
- NGN-401 continues to be generally well-tolerated in Embolden, with no treatment-related SAEs or DLTs as of the data cutoff date of June 16, 2026

Neurogene management and Dr. Suter will discuss these results during a live webcast today, June 29, 2026, at 8:00 a.m. ET. The live webcast presentation will be accessible from the Investor Relations section of the Company's website under Events, where a replay of the event will also be available for a limited time.

These data, as well as a poster presentation on the Rett Syndrome Natural History Study analysis to assess treatment effect in the Embolden trial, will also be presented at the 2026 International Rett Syndrome Foundation (IRSF) Rett Syndrome Scientific Meeting taking place in Prior Lake, Minn., June 29 – July 1, 2026.

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. Data from the Phase 1/2 trial (as of June 16, 2026) have shown that participants experienced multidomain, durable gains with continued developmental milestone 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 results from the NGN-401 Phase 1/2 clinical trial to participants in the Embolden registrational trial; 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; the response rate, expected durability and deepening of clinical data results from our NGN-401 clinical trial; expected timing and availability for release of topline data from our Embolden clinical trial of NGN-401 for Rett syndrome; the potential for success of the Embolden trial; the clinical benefit of delivering NGN-401 via intracerebroventricular administration; and 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. 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 risks and uncertainties identified under the heading "Risk Factors" included in Neurogene's Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, filed with the Securities and Exchange Commission ("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.

Media Contact:
Mike Devine
Executive Director, Corporate Communications
michael.devine@neurogene.com

Investor Contact:
Lina Li
Executive Director, Investor Relations
lina.li@neurogene.com

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NEUROGENE
Restarting Development in Rett Syndrome
NGN-401 for Rett Syndrome
Phase 1/2 Update
June 29, 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 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 results from the NGN-401 Phase 1/2 clinical trial to participants in the Embolden™ registrational clinical trial of NGN-401; the likelihood of gaining approval of NGN-401 from the U.S. Food and Drug Administration (FDA) or any other regulator; trial designs and clinical development plans for the Embolden trial; the response rate, expected durability and deepening of clinical data results from the NGN-401 clinical trials; expected timing and availability of topline results from the Embolden registrational trial; the timing of a potential submission of a Biologics License Application (BLA) to the FDA based on the results of the Embolden trial and the ability of the Embolden registrational trial to support planned BLA submissions; the potential for NGN-401 to be a best-in-class or first-in-class gene therapy for Rett syndrome; the potential for success of the Embolden registrational clinical trial of NGN-401 for Rett Syndrome; the potential real-world impact of NGN-401; 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, 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

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Today's Speakers

Rachel McMinn, Ph.D.
Founder and Chief Executive Officer, Neurogene

Bernhard Suter, M.D.
Medical Director, Blue Bird Circle Rett Center, Texas Children's Hospital
Associate Professor of Pediatrics and Neurology, Baylor College of Medicine

Julie Jordan, M.D.
Chief Medical Officer, Neurogene

NEUROGENE

Introduction

Rachel McMinn, Ph.D.
Founder and Chief Executive Officer, Neurogene

No Approved Disease-Modifying Therapies for Rett Syndrome
NGN-401 Gene Therapy Targets Root Cause

The Unmet Need

- Developmental regression begins at **6–18 months**
- Previously acquired **milestones are lost**
- Development plateaus at **age ~3**
- Milestone acquisition is **rare post-regression**
- Patients require lifelong, **24/7 care**
- No approved therapies to address underlying cause, current options limited to symptom management

The Market

15,000–20,000
patients — US, EU & UK

1 in 10,000
female births | ~175–180 new cases/yr (U.S.)

U.S. Real-World Data – ~6,000 Diagnosed

52% Pediatric	48% Adult

All living with profound unmet need

The Opportunity

- The Rett syndrome community is increasingly seeking therapies that can deliver meaningful and durable functional improvements
- Patient population across all age groups creates the potential for broad adoption of transformative therapies

Rett syndrome is a lifelong disease. The opportunity spans every age group and remains entirely unaddressed at the genetic level

NGN-401 is designed to deliver functional *MECP2* — targeting the root cause with the potential to restart typical developmental progression

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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):347–352.
WW incidence estimate based on published incidence rates; Pini G, et al. Orphanet Journal of Rare Diseases (2016) 11:132.
Cheng et al., BMC Neurology, 2023 (IQVIA Claims, N=5,940)

NGN-401 Phase 1/2 Trial Update: Clinically Meaningful, Durable Improvement Across Key Rett Syndrome Domains, Deepens Over Time

100%

of Phase 1/2 participants (N=10) gained ≥1 developmental milestone and improved on CGI-I

80% met Embolden composite responder definition at 12 months (8 of 10)

47

total milestones gained

4.7 average milestones per participant

30

months of **continued improvement post-dose**

no plateau observed – no milestones lost

Restarted Development

Milestones acquired in a **developmentally ordered stepwise sequence** — suggesting a **restart of developmental trajectory**

Real-World Impact

- ↑ **Gains across daily living**
- ↑ **Increased independence**
- ↓ **Reduced caregiver burden**

Safety: 1E15 vg dose generally well-tolerated | **No Treatment-Related SAEs or DLTs** in Embolden

Embolden™ Registrational Trial: All 25 participants successfully dosed – overenrolled based on demand from the Rett syndrome community

Topline Results
Expected 2H 2027

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As of data cutoff date of June 16, 2026
CGI-I = Clinician Global Impression-Improvement; DLT = Dose limiting toxicity; SAE = Serious adverse event

Long-term Phase 1/2 Data Demonstrate Broad, Consistent Multidomain Developmental Gains that Support Registration Regardless of Age, Disease Severity or Genotype

- 100% of Phase 1/2 participants (N=10) gained ≥1 developmental milestone and improved on CGI-I at ≥12 months
- Rapid onset of clinical response, with median first improvement observed at 2 months post-treatment, followed by early emergence of developmental milestones
- Durable, deepening treatment effect — developmental milestones continued to accumulate over time
  - Developmental milestone gains increased by **95% from 6 to 12 months** and **147% from 6 to ≥12 months**
  - Durable treatment effect, with **no plateau** and **no loss of milestones in any participant** through 30 months of follow-up
- Broad, multidomain functional impact demonstrated consistently across core disease domains, regardless of age, disease severity or genotype
  - **7 of 10 participants gained ≥2 developmental milestones**
  - **7 of 10 participants gained milestones in ≥2 core Rett syndrome domains**
  - Durable, multidomain gains drive increased independence in activities of daily living, reduced caregiver burden and enhanced social engagement
- Robust, clinically meaningful responses at 6 months, 12 months and beyond support clear path to potential BLA
  - Average milestones per participant showed **robust response: 1.9 at 6 months**, **3.7 at 12 months, deepening to 4.7 at ≥12 months**
  - **Embolden overenrolled by 25%** (N=25), across broad age range, strengthening statistical power and potential for broader label
  - Phase 1/2 data **exceeds** Embolden's minimum success **threshold by 2.4x**
- No new treatment-related SAEs and no DLTs observed in any participants, with all participants ≥12 months of follow-up

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Phase 1/2 Data Update

NGN-401 Clinical Study Design for Treatment of Rett Syndrome

Phase 1/2 converted into a single registrational study

Study Design

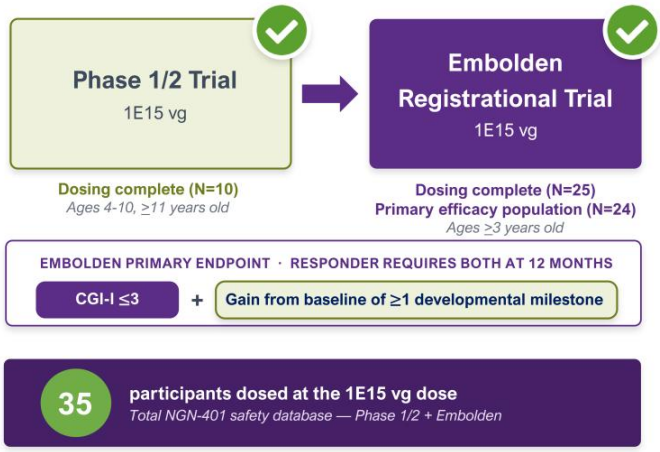
- Baseline-controlled, open-label, multicenter, single-arm pivotal trial (13 US sites)
- Evaluates efficacy, safety & tolerability of one-time ICV-delivered NGN-401

Population Eligibility Criteria

- Females with genetically confirmed classic Rett syndrome
- Post-regression (≥6 months since last skill loss); CGI-S 4–6 at screening

Key Clinical Assessments

- CGI-I and CGI-S with Rett-specific anchors
- Developmental milestones
- RSGMS and RSHFS



35 **participants dosed at the 1E15 vg dose**
Total NGN-401 safety database — Phase 1/2 + Embolden

CGI-S = Clinical Global Impression–Severity; ICV = intracerebroventricular; RSGMS = Rett Syndrome Gross Motor Scale; RSHFS = Rett Syndrome Hand Function Scale; vg = vector genomes

28 Clearly Defined Developmental Milestones: Derived from Natural History Study & Key Component of Primary Endpoint

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 (i.e., walk with support)
- 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)

Rigorous Embolden Milestone Criteria Evaluation Applied to Phase 1/2 Data

Utilizing the same standardized assessment criteria enables the Phase 1/2 developmental milestone data to be comparable to the Embolden definition

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

Baseline Characteristics of the Phase 1/2 Participants

	Pediatric Cohort (N=8)								Adult / Adolescent Cohort (N=2)	
	Pt:1	Pt:2	Pt:3	Pt:4	Pt:5	Pt:6	Pt:7	Pt:8	Pt:9	Pt:10
Age at Dosing (Years)	7	4	6	7	6	4	6	8	18	14
Baseline CGI-S Score	4 Moderately Ill	5 Markedly Ill	5 Markedly Ill	5 Markedly Ill	6 Severely Ill	5 Markedly Ill	4 Moderately Ill	4 Moderately Ill	4 Moderately Ill	5 Markedly Ill
Genetic Variant Severity	Mild	Severe	Severe	Severe	Severe	Moderate	Mild-Moderate	Mild-Moderate	Severe	Severe
Follow-up Period (Months)	30	24	24	24	18	12	12	12	15	12

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As of data cutoff date of June 16, 2026
Pt = Participant

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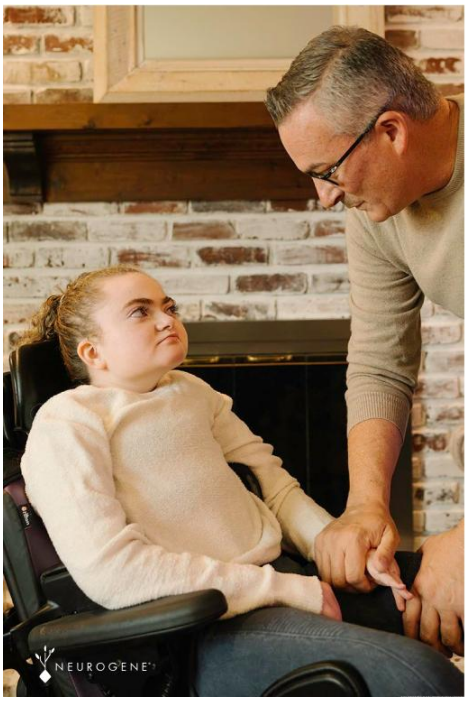
Phase 1/2 Data Update:
Restarting Development in Long-Term NGN-401-Treated Participants

Bernhard Suter, M.D.

Medical Director of Blue Bird Circle Rett Center at Texas Children's Hospital, Associate Professor of Pediatrics and Neurology, Baylor College of Medicine

Principal Investigator in NGN-401 Clinical Trial



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

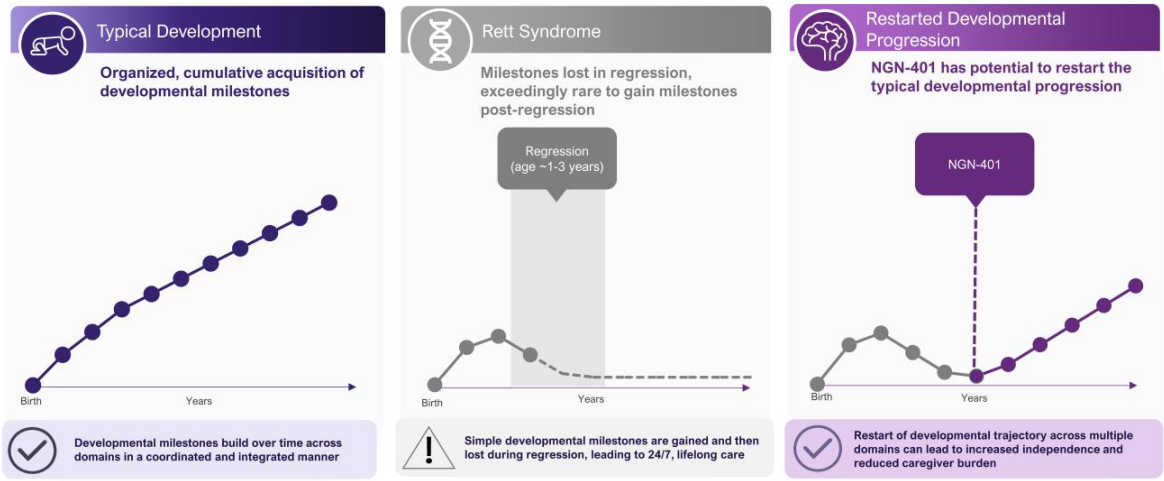
Hallmark features:

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

Living with Rett Syndrome

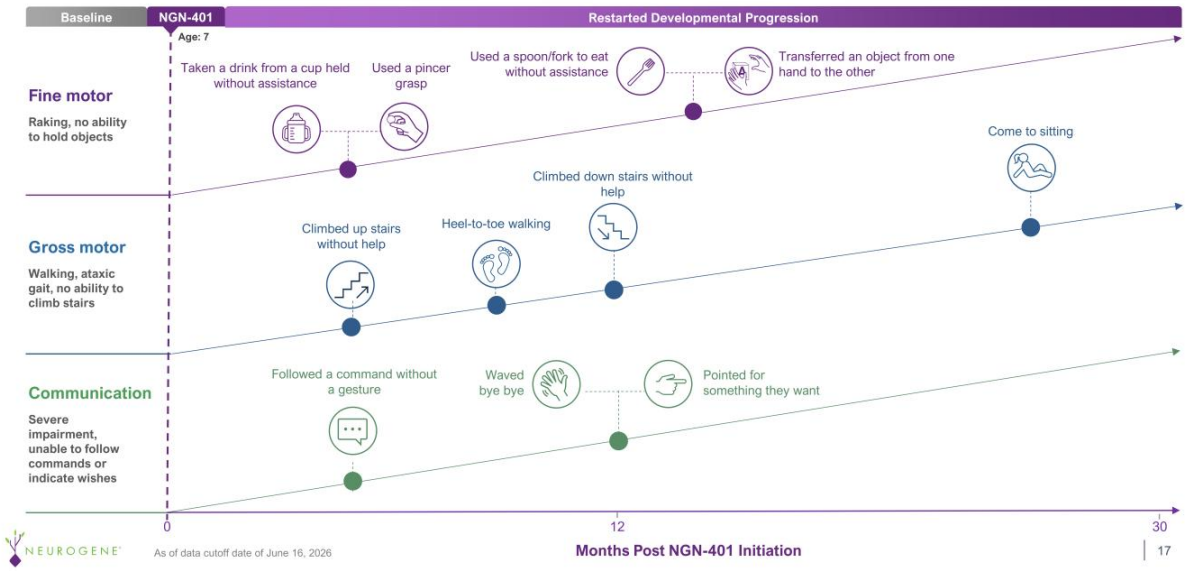
Kendall & Kati

Restarting Developmental Progression is the Goal for a One-time Gene Therapy



Frankenburg et al., Denver II Technical Manual May D, *et al. J Neurodev Disord.* 2024;16(1):42
Graphs for illustrative purposes only

Pt:1 Developmental Milestones Gained in Ordered, Developmental Progression



NEUROGENE

As of data cutoff date of June 16, 2026

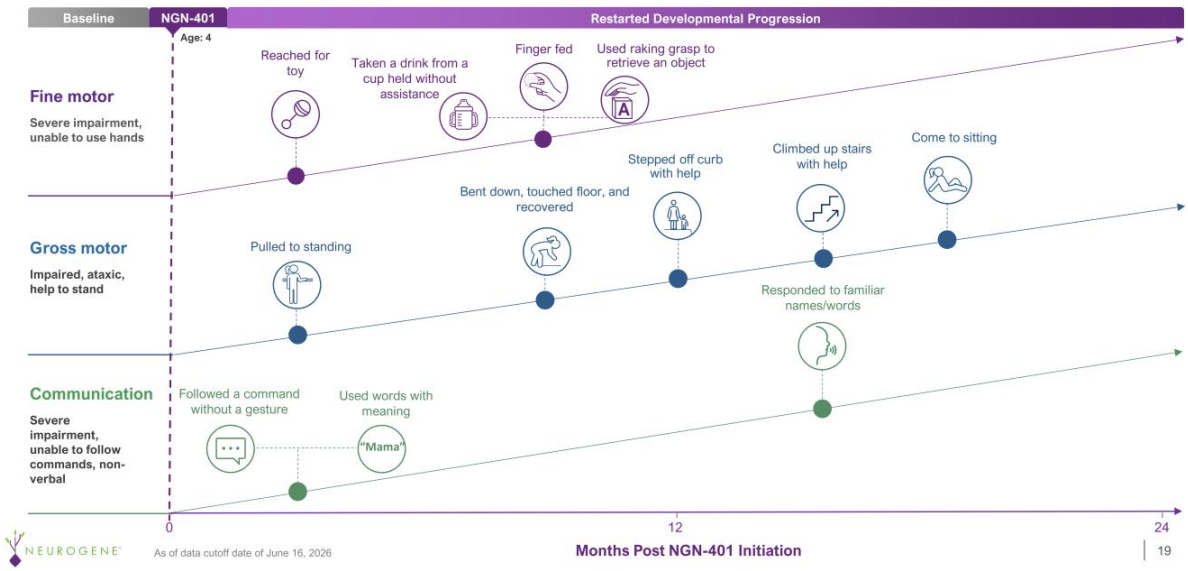
Pt:1 Multidomain Improvements Led to New Abilities Beyond Developmental Milestones

At baseline (Age 7): **Impaired hand use,** Raking, no ability to hold objects | **Gross motor impairment,** ataxic, unable to climb stairs | **Severe communication impairment,** unable to follow commands

Moves more independently at home	**Joins family outings**	**Navigates the world independently**	**Follows multi-step directions**	**Follows instructions to pick the right colors**	**Greets family in context**
					
Gets in/out of bathtub, on/off furniture & up/down stairs, without help	Shops with Mom, carrying the basket with both hands	Climbs in & out of the car & shuts the door herself	Carries her backpack, takes the stairs, & closes the door – following directions in 2 languages	Uses hands to select correct colors when asked	Waves hello to greet people on video calls

Enhanced Independence and Reduced Caregiver Burden Post-NGN-401

Pt:2 Developmental Milestones Gained in Ordered, Developmental Progression



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As of data cutoff date of June 16, 2026

Pt:2 Multidomain Improvements Led to New Abilities Beyond Developmental Milestones

At baseline (Age 4):	**Severe impairment,** unable to use hands	**Gross motor impairment,** ataxic, needs help to stand	**Severe communication impairment,** unable to follow commands

Less caregiver assistance at mealtime

Self-feeds and holds own drinks

Moves more independently with less supervision

Bends down to pick up toys & blankets without help

Connects with family

Turns when called, says words with meaning & follows instructions

Enhanced Independence and Reduced Caregiver Burden Post-NGN-401

As of data cutoff date of June 16, 2026

Pt:3 Multidomain Improvements Led to New Abilities Beyond Developmental Milestones

At baseline (Age 6):	**Impaired hand use,** raking grasp	**Severe gross motor impairment,** cannot sit/stand/walk independently	**Severe communication impairment,** unable to follow commands

Uses both hands to follow instructions

Follows directions in two languages and chooses the correct puzzle piece

Stands and moves with less help

Requires less physical support to stand and move

Feeds herself

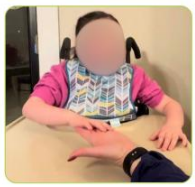
Self-feeds using a pincer grasp

Enhanced Independence and Reduced Caregiver Burden Post-NGN-401

As of data cutoff date of June 16, 2026

Pt:4 Multidomain Improvements Led to New Abilities Beyond Developmental Milestones

At baseline (Age 7): **Impaired hand use,** raking grasp | **Severe gross motor impairment,** cannot sit/stand/walk independently | **Severe communication impairment,** unable to follow commands

Eats on her own	Sits up by herself	Controls her space	Plays with Mom with both hands	Joins in family play	Makes her own needs known	Connects with family
						
Feeds herself with a regular spoon — easier, more independent mealtimes	Pushes herself up from lying down, moving through her day with more freedom	Turns the lights on and off herself, taking charge of her surroundings	Catches a ball with both hands and plays back-and-forth with others	Plays with balloons at a family celebration, demonstrating her new hand use	Tells her caregiver what she wants more reliably using her communication device	Shares more moments with family, including giving "high fives"

Enhanced Independence and Reduced Caregiver Burden Post-NGN-401

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Thank you to all participants and their families and caregivers!

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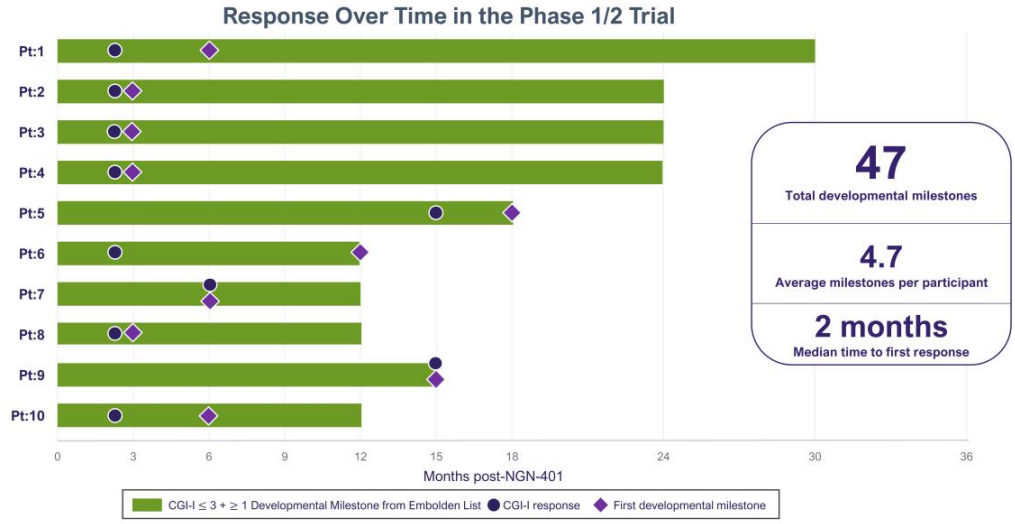
Phase 1/2 Data Update

Julie Jordan, M.D.
Chief Medical Officer

Rapid Response Observed Post NGN-401 that Deepened Over Time



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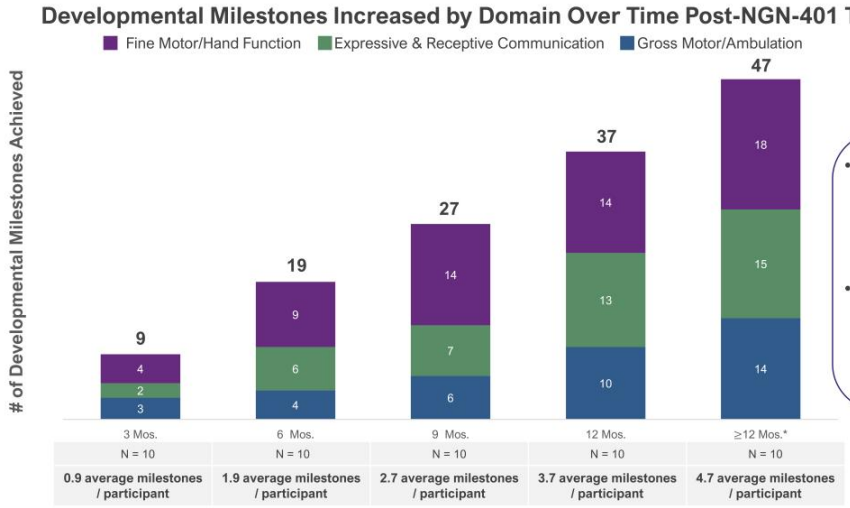
As of data cutoff date of June 16, 2026
Four previously disclosed developmental milestones included are derived from other validated scales and are not included in the Embolden list:
Pt. 1 - Heel to toe walking – 9 mos.; Pt. 2 – Bent down, touched floor and recovered – 9 mos.; Stepped off curb with help – 12 mos.; Pt. 8 – Bent down, touched floor and recovered – 3 mos.

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



- **Milestones increased by 95% from 6 to 12 months and 147% from 6 to ≥12 months post-NGN-401**
- **7 of 10 participants gained:**
  - **≥2 milestones**
  - **Milestones in ≥2 core domains**

As of data cutoff date of June 16, 2026
Four previously disclosed developmental milestones included are derived from other validated scales and are not included in the Embolden list:
Pt. 1 – Heel to toe walking – 9 mos.; Pt. 2 – Bent down, touched floor and recovered – 9 mos.; Stepped off curb with help – 12 mos.; Pt. 8 – Bent down, touched floor and recovered – 3 mos.
*N=6 participants with data > 12 mos. post-NGN-401 and N=4 participants with 12-month data

Milestones Gained from Embolden List Across 10 Phase 1/2 Participants Treated with NGN-401

21 of 28 Total Milestones Gained from Embolden Milestone List

7/7

Fine Motor / Hand Function

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

Participating in self care and increasing independence — playing games with family, feeding themselves, drinking from a cup

7/13

Gross Motor / Ambulation

- Sat without support when placed
- Come to sitting
- Pulled to standing
- Cruised / walk with support
- Climbed up stairs with help
- Climbed up stairs without help
- Climbed down stairs without help

Moving more independently — sitting up, standing, and walking — easing everyday care for families

7/8

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
- Used words with meaning
- Spoken in phrases

Connecting with the people around them — understanding others, and making their wants and feelings known

NEUROGENE As of data cutoff date of June 16, 2026

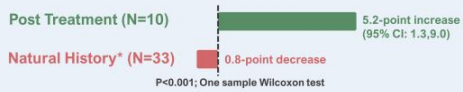
RSGMS Phase 1/2 Data Showed Meaningful Gains in Gross Motor Function

Overview of Rett Syndrome Gross Motor Scale (RSGMS)

Validated clinical tool that assesses 15 key gross motor abilities specifically in individuals with Rett syndrome

Increase in 3+ points is considered a meaningful gain in motor ability

Mean RSGMS Total-Score Gain Exceeded Natural History Normalized to One Year Rate



Real World Impact of RSGMS Gains: Examples of Reduced Caregiver Burden

7 unable to perform independently at baseline

SIT TO STAND

Reduces physical burden on caregiver, enhances independence

6 ambulatory unable to perform independently at baseline

STAND UP FROM FLOOR

Complex motor planning allowing for greater autonomy and reduced caregiver physical burden

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As of data cutoff date of June 16, 2026

* Bisgaard AM *et al. Front Neurol* 2026; 16: 1702703

IRSF Scientific Meeting 2026

RSHFS Phase 1/2 Data Showed Meaningful Gains in Hand Function

Overview of Rett Syndrome Hand Function Scale (RSHFS)

Validated clinical tool that assesses levels of hand function specific to Rett syndrome

Increase in 1+ points is considered a meaningful gain in hand function

Mean RSHFS Total-Score Gains Exceed Natural History

Real World Impact: More ability to play, less reliance on caregiver at mealtime, participation in family routines

10/10 improved

N=8/10 improved in total score, N=1 improved grasp, N=1 acquired new hand function developmental milestone

GRASPING, PICKING UP, HOLDING LARGE OR SMALL OBJECTS

Enhances ability to self-feed, increases independence

1.8 point gain per hand

7/9 bilateral improvement

N=9 at baseline had significant hand function impairment

MORE COMPLEX ACTIVITIES

Enables using utensils, more coordination to play, interact, and be independent

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As of data cutoff date of June 16, 2026

*Downs J *et al. J Pediatr* 2021; 237: 244–249; PMID: 34214590

IRSF Scientific Meeting 2026

NGN-401 Remains Generally Well-Tolerated at the 1E15 vg Dose Level in the Phase 1/2 Trial

Phase 1/2 Trial	1E15 vg Dose Total N = 10	
	N	Events
TEAEs related to NGN-401	9	68
Serious TEAEs Unrelated to NGN-401	3	6
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 common related TEAEs: mild ALT/AST elevation
- No new treatment-related SAEs reported since last data cutoff date (October 2025)
  - Two previously disclosed Grade 2 SAEs in Pt:5 resolved
- No cases of hemophagocytic lymphohistiocytosis (HLH)
- No intracerebroventricular (ICV) procedure-related AEs
- No signs or symptoms of MeCP2 overexpression
- Seizures have remained well controlled following NGN-401

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As of data cutoff date of June 16, 2026
TEAE = Treatment emergent adverse event

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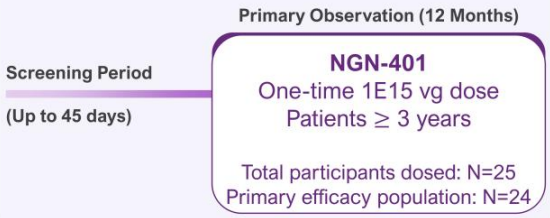
Embolden
A Clinical Study for Rett Syndrome

Embolden Registrational Trial

Dosing Completed in Embolden Registrational Trial to Support Planned BLA Submission

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**

33% response rate, or 8 of 24 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**

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Immunosuppression: Steroid prophylaxis during first 90 days, followed by taper
BLA = Biologics license application

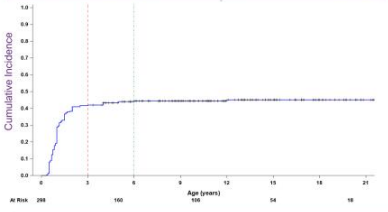
Natural History Study Cumulative Incidence Model Shows Beyond Age 3, Milestone Gains Are Rare with Minimal Difference at Age 6

Likelihood of gaining a milestone at ≥ 3 years of age is extremely low, based on RNHS analysis

Fine Motor

Example Milestone: Taken a drink from a cup held without assistance

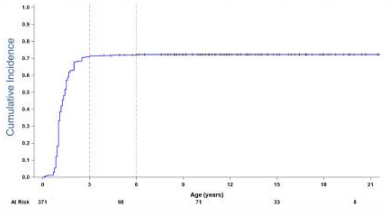
% ever gained/regained: **46%** | At ≥3 years: **4.2%** | At ≥6 years: **1.3%**



Gross Motor

Example Milestone: Cruised around furniture or holding on to someone (i.e., walk with support)

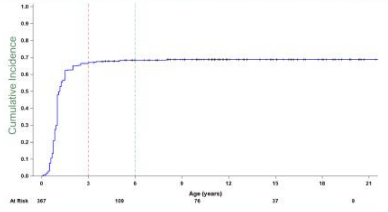
% ever gained/regained: **72%** | At ≥3 years: **1.5%** | At ≥6 years: **0.3%**



Communication

Example Milestone: Used words with meaning

% ever gained/regained: **69%** | At ≥3 years: **2.5%** | At ≥6 years: **0.4%**



Phase 1/2 trial participants were evaluated using the Embolden Developmental Milestone List, and in every age group, NGN-401-treated participants gained multiple milestones

Ex: Participant **Age 4**
10 milestones gained
Age-matched RNHS analysis cumulative incidence rate: **0.3-7.9%**

Ex: Participant **Age 7**
10 milestones gained
Age-matched RNHS analysis cumulative incidence rate: **0.0-2.0%**

Ex: Participant **Age 14**
2 milestones gained
Age-matched RNHS analysis cumulative incidence rate: **0.0%**

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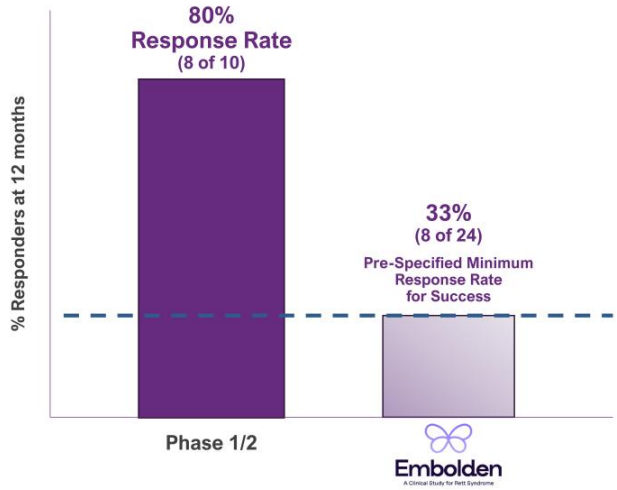
As of data cutoff date of June 16, 2026
IRSF Scientific Meeting 2026

Compelling Clinical Data Strengthen Confidence in Potential Embolden Registrational Success



- Phase 1/2 response rate exceeds the success threshold defined for Embolden by 2.4x
- NGN-401 has been generally well-tolerated in Embolden
- No treatment-related SAEs or DLTs in Embolden trial

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Advancing NGN-401 Towards Commercialization

Rachel McMinn, Ph.D.
Founder and Chief Executive Officer, Neurogene

Robust, Clinically Meaningful Responses Across Participants Support Potential Market Leading Therapy for Rett Syndrome

NGN-401 Phase 1/2 Data (N=10)		
Developmental Milestones	% of participants gained ≥1 developmental milestone at ≥12 months	**100%**
	Average milestones gained per participant	**4.7**
	Total Participants achieving: - ≥2 milestones - Response on ≥2 domains	**7 out of 10**
	Average # milestones at: - 6 months - 12 months - ≥12 months	 **1.9** **3.7** **4.7**
	% increase from: - 6 to 12 months - 6 to ≥12 months	 **95%** **147%**
	# of milestones lost in any participant	**0**
Embolden Composite	% meeting rigorous composite responder definition (CGI-I ≤3 + ≥1 milestone)	**80%**
Rapid Response	Median time to first response observed post treatment	**2 months**
RSGMS RSHFS	Independent, quantitative validated scales of function in Rett syndrome compared to natural history data	• 5.2-point increase vs. 0.8-point decrease (p<0.001) • 100% vs. 15% improved in hand function (p<0.001)
No new treatment-related SAEs and no DLTs observed in any participants, with all participants ≥12 months of follow-up		

Phase 1/2 shows robust response across key Embolden endpoints having the potential to drive a differentiated label

Embolden fully dosed, topline data expected 2H 2027

As of data cutoff date of June 16, 2026

NGN-401 is Poised to Transform Treatment for Rett Syndrome

TODAY

- **Compelling Clinical Data Showed NGN-401 Restarts the Development Trajectory**
- **Completed Dosing in Embolden Registrational Trial**
- **Early Commercial-Readiness Underway**
  - **Chief Commercial Officer Added to Senior Leadership Team**

UPCOMING ANTICIPATED MILESTONES

- **Start PPQ Campaign Mid-2026**
- **Topline Data from Embolden in 2H 2027**
- **BLA Submission for NGN-401**
- **Continue 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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Thank you to all participants, caregivers, families, investigators, clinical site coordinators, study coordinators, site staff and the Rett syndrome community for your trust, partnership and ongoing support of the development of NGN-401!