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): November 9, 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 November 9, 2026, Neurogene Inc. (the "Company") issued a press release announcing financial results for the quarter ended September 30, 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 November 9, 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 November 9, 2026
99.2	Corporate Presentation (November 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: November 9, 2026

By: /s/ Christine Mikail

Name: Christine Mikail

Title: President, Chief Financial Officer

Neurogene Reports Third Quarter 2026 Financial Results and Highlights Recent Updates

Topline data from Embolden™ registrational trial anticipated in 2H 2027

Presented new preclinical data highlighting the purposeful design of NGN-401 for Rett syndrome, including rapid onset and durable expression of full-length MECP2 *transgene with single-stranded AAV construct*

NGN-401 at the 1E15 vg dose remains generally well-tolerated (N=35) as of data cutoff date of November 8, 2026

On track to complete PPQ runs by year-end 2026

Strong cash position provides runway into first quarter of 2029

NEW YORK – Nov. 9, 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 third quarter 2026 financial results and highlighted recent corporate updates.

"During the third quarter, we continued to execute across all aspects of our NGN-401 program as we advance toward potential commercialization," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "With topline data from the Embolden registrational trial expected in the second half of 2027, we are progressing key BLA and commercial-readiness activities, including PPQ runs that we believe remain on track for completion this year. We are also continuing to strengthen our engagement with the Rett syndrome community and the broader medical community to increase awareness and understanding of NGN-401 and the growing body of data supporting its potential as a best-in-class, one-time treatment for Rett syndrome. Together, these efforts are aimed at positioning us for a successful launch, if approved."

Third Quarter 2026 and Recent Highlights

NGN-401 Gene Therapy for the Treatment of Rett Syndrome

- Anticipate reporting topline data from the Embolden™ registrational trial in the second half of 2027
    - As previously announced, the Embolden trial was overenrolled due to strong demand from the Rett syndrome community. Neurogene elected to include all 25 participants dosed in the primary efficacy analysis. The update to the statistical analysis plan was aligned with the FDA through the START Pilot Program. The

corresponding threshold for success to reject the null hypothesis is 9 of 25 participants (36%). All other previously aligned key elements of the trial design remain unchanged.

- Presented an encore of the positive long-term Phase 1/2 data, as of the data cutoff date of June 16, 2026, at the 55th Child Neurology Society Annual Meeting
  - Phase 1/2 data demonstrated developmental milestones were gained in a progressive, stepwise sequence, suggesting a restart of development post-treatment
- Presented data at the European Society of Gene and Cell Therapy 2026 Annual Congress that highlighted the key differentiating design elements of NGN-401 for Rett syndrome:
  - New preclinical data showed rapid onset of action and durable expression of MeCP2 protein following single-stranded AAV (ssAAV) delivery of the NGN-401 construct; the data provided additional evidence that self-complementary AAV does not offer a meaningful advantage in onset of action in the treatment of Rett syndrome
    - NGN-401 was designed to use ssAAV to accommodate the fully functional, full-length *MECP2* transgene rather than a minigene
  - New in vivo evaluation of transgene regulation technologies demonstrated that miRNA-based auto-regulatory technology was not responsive to endogenous MeCP2 levels on a cell-by-cell basis; the data showed expression in both MeCP2-null and MeCP2-expressing neurons
  - An encore presentation supported the therapeutic rationale for the intracerebroventricular (ICV) administration of central nervous system (CNS)-targeted gene therapy, including NGN-401; ICV administration is a standard neurosurgical procedure performed thousands of times per year, and preclinical data demonstrated broader CNS biodistribution to brain regions central to Rett syndrome pathophysiology, as compared with intrathecal lumbar administration
- Partnered with the International Rett Syndrome Foundation to host a webinar focused on educating the Rett syndrome community about NGN-401
- NGN-401 at the 1E15 vg dose has been generally well-tolerated in the Phase 1/2 trial and Embolden (N=35) as of November 8, 2026
- On track to complete Process Performance Qualification (PPQ) runs by the end of 2026 to support the planned Biologics License Application (BLA) submission

Key Anticipated NGN-401 Milestones

- Report topline data from Embolden registrational trial in the second half of 2027
- Complete BLA-enabling PPQ runs by year-end 2026
- Continue commercial-readiness activities

Upcoming Events

- Guggenheim 3rd Annual Healthcare Innovation Conference: Management will participate in a fireside chat at 10:30 a.m. ET on November 10 and participate in investor meetings

- Stifel 2026 Healthcare Conference: Management will participate in a fireside chat at 10:40 a.m. ET on November 11 and participate in investor meetings

Third Quarter 2026 Financial Results

- **Cash, Cash Equivalents and Short-Term Investments**: Cash, cash equivalents and short-term investments as of September 30, 2026 were $359.7 million and are expected to fund planned operations into the first quarter of 2029.
- **Research & Development (R&D) Expenses**: R&D expenses were $23.5 million for the three months ended September 30, 2026, compared to $17.2 million for the three months ended September 30, 2025. The increase in R&D expenses for the three months ended September 30, 2026 was primarily driven by higher costs associated with the development of NGN-401, including Rett syndrome clinical trial activities and chemistry, manufacturing and controls (CMC) activities supporting the NGN-401 program. The increase also reflected higher employee-related expenses due to an increase in R&D headcount and increased non-cash stock-based compensation expenses. The increase was partially offset by lower spending on the CLN5 Batten disease program.
- **General & Administrative (G&A) Expenses**: G&A expenses were $8.9 million for the three months ended September 30, 2026, compared to $6.6 million for the three months ended September 30, 2025. The increase in G&A expenses was primarily driven by higher non-cash stock-based compensation expense, driven by an increase in headcount as well as an increase in performance-based stock unit awards following achievement of specified corporate milestones. Additional increases were driven by corporate and pre-commercial activities, higher employee-related expenses and professional fees.
- **Net Loss**: Net loss was $29.0 million for the three months ended September 30, 2026, compared to $21.0 million for the three months ended September 30, 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. 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 interim results from the NGN-401 Phase 1/2 clinical trial to other participants; 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 expected timing and successful completion of the Company's PPQ campaign and other CMC activities to support a planned BLA submission; 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 topline data from the Company's registrational trial of NGN-401; the potential for success of the Embolden registrational clinical trial of NGN-401 for Rett syndrome; the extent to which preclinical data, including data regarding the onset and durability of transgene expression, transduction efficiency and biodistribution, may be predictive of clinical trial results; clinical benefit of delivering NGN-401 via intracerebroventricular administration; expected future interactions with or positions of the FDA or foreign regulatory authorities, 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 EMA's PRIME designation; plans for a BLA submission for NGN-401; 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 or the Embolden registrational clinical trial of NGN-401 for the treatment of Rett syndrome; the risk that preclinical data or interim clinical results may not be predictive of results of the Embolden registrational trial or of future clinical experience; the risk that the Company may not be able to report data on the predicted timeline; the risk that the Company may not complete its PPQ campaign on the anticipated timeline or at all, or may encounter other manufacturing or CMC challenges; risks related to the Company’s ability to obtain regulatory approval for, and ultimately commercialize, its product candidates, including NGN-401; 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 June 30, 2026, filed with the SEC on August 11, 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)

	September 30, 2026	December 31, 2025
Assets		
Cash and cash equivalents	$ 78,942	$ 103,845
Short-term investments	280,776	165,168
Other current assets	3,021	2,757
Non-current assets	15,162	16,834
Total assets	$ 377,901	$ 288,604
Liabilities		
Current liabilities	$ 18,163	$ 16,411
Non-current liabilities	4,944	7,306
Total liabilities	23,107	23,717
Stockholders' equity	354,794	264,887
Total liabilities and stockholders' equity	$ 377,901	$ 288,604

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating expenses:				
Research and development expenses	$ 23,522	$ 17,241	$ 74,140	$ 54,372
General and administrative expenses	8,854	6,557	28,255	21,427
Total operating expenses	32,376	23,798	102,395	75,799
Loss from operations	(32,376)	(23,798)	(102,395)	(75,799)
Other income, net	3,353	2,845	7,950	10,183
Net loss	$ (29,023)	$ (20,953)	$ (94,445)	$ (65,616)
Per share information:				
Net loss per share, basic and diluted	$ (1.05)	$ (0.99)	$ (3.90)	$ (3.11)
Weighted-average shares of common stock outstanding, basic and diluted	27,695,698	21,168,431	24,204,232	21,073,996

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NEUROGENE
EVERY BREAKTHROUGH
BEGINS WITH BELIEF
Corporate Presentation
November 2026

Disclaimer

Forward Looking Statements

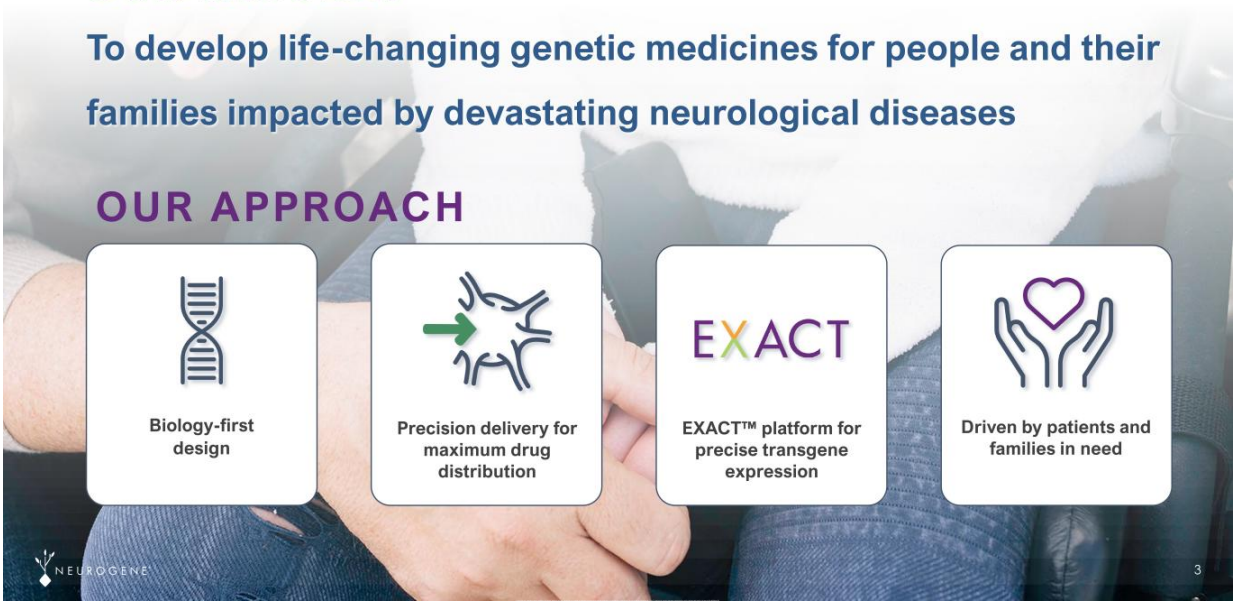
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These statements may discuss goals, intentions and expectations as to future plans, trends, events, results of operations or financial condition, or otherwise, based on current expectations and beliefs of the management of Neurogene, as well as assumptions 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 success of the Embolden registrational clinical trial of NGN-401 for Rett syndrome; the response rate, expected durability and deepening of clinical data results from the NGN-401 clinical trials; the potential for future approval for commercialization of NGN-401 as a treatment for Rett syndrome and the speed with which any such approval might be obtained; commercial launch readiness for NGN-401; 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 timing and the expected successful completion of our Process Performance Qualification (PPQ) activities and other chemistry, manufacturing and controls (CMC) requirements to support a planned BLA submission; the applicability of reported results from the NGN-401 Phase 1/2 clinical trial to other participants or potential participants; any extrapolation of interim trial results on 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™ registrational clinical trial of NGN-401 for Rett syndrome; expected timing for release of topline data from the Embolden registrational trial of NGN-401; the extent to which preclinical data, including data regarding the onset and durability of transgene expression, transduction efficiency and biodistribution, may be predictive of clinical trial results; 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; 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; 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 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. 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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
- Completed dosing (N=25) in Embolden, exceeding target dosing due to high demand from Rett syndrome community

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

- Topline data from Embolden **anticipated in 2H 2027**
- Completion of BLA-enabling Process Performance Qualification (PPQ) runs **expected by year-end 2026**

Neurogene is Positioned for Significant Value Inflection with Multiple Upcoming Catalysts

NEUROGENE

NGN-401 FOR RETT SYNDROME

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

NEUROGENE

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: 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

NEUROGENE

NGN-401 CLINICAL PROGRAM

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, with no cases of HLH in the Phase 1/2 or Embolden

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

Topline Results
Expected 2H 2027

NEUROGENE

Efficacy data as of data cutoff date of June 16, 2026
1E15 vg dose generally well-tolerated as of November 8, 2026
CGI-I = Clinician Global Impression-Improvement; HLH = hemophagocytic lymphohistiocytosis

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 >2x**
- No new treatment-related SAEs and no DLTs observed in any participants, with all participants ≥12 months of follow-up

NGN-401 Clinical Study Design for Treatment of Rett Syndrome

Phase 1/2 converted into a single registrational study

Study Design

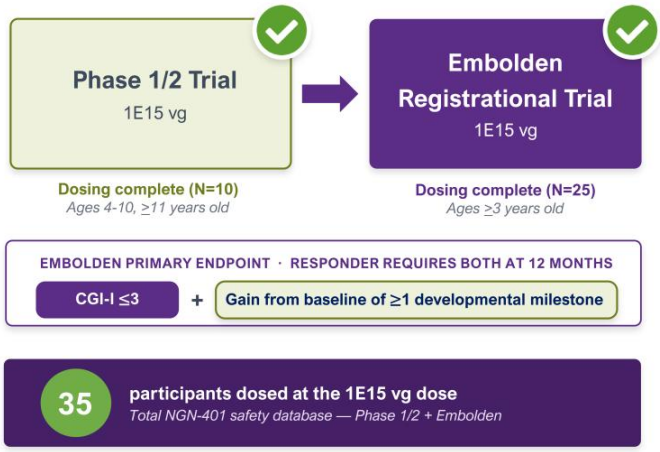
- Baseline-controlled, open-label, multicenter, single-arm pivotal trial (13 US sites)
- Evaluates efficacy, safety & tolerability of one-time ICV-delivered NGN-401

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



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

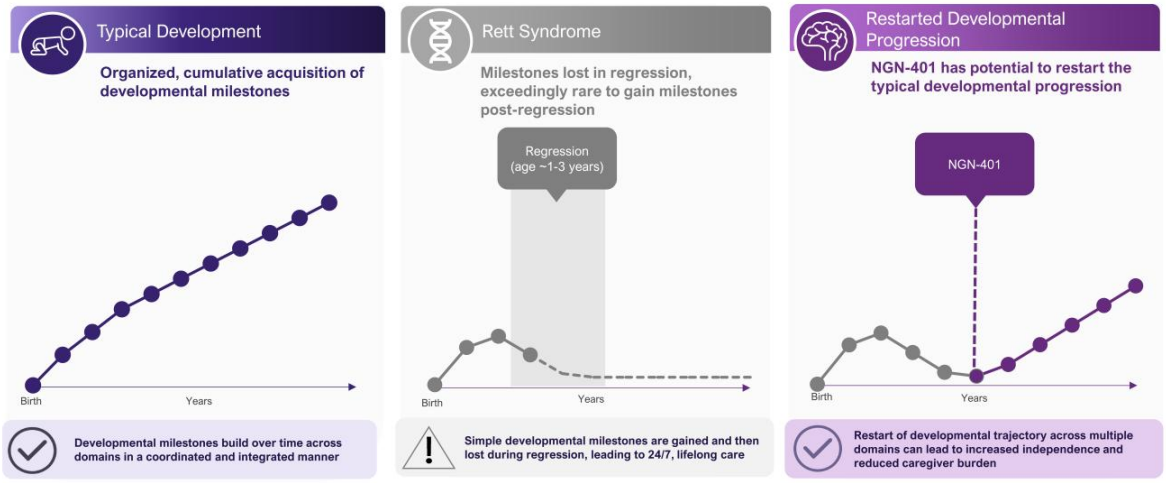
Baseline Characteristics of the Phase 1/2 Participants

	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

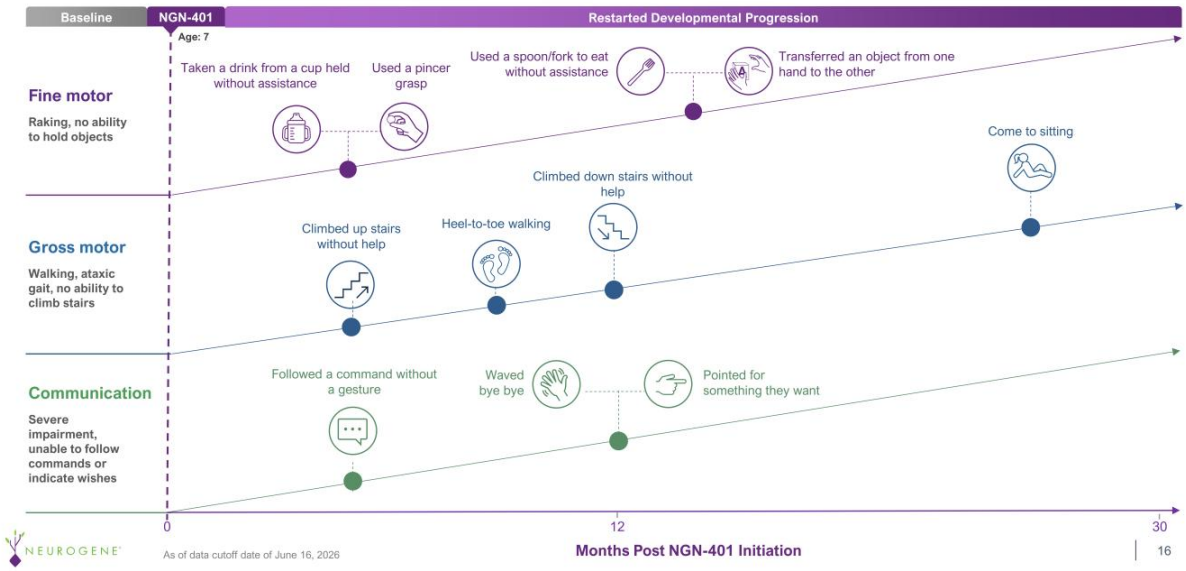
Restarting Developmental Progression is the Goal for a One-time Gene Therapy



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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



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

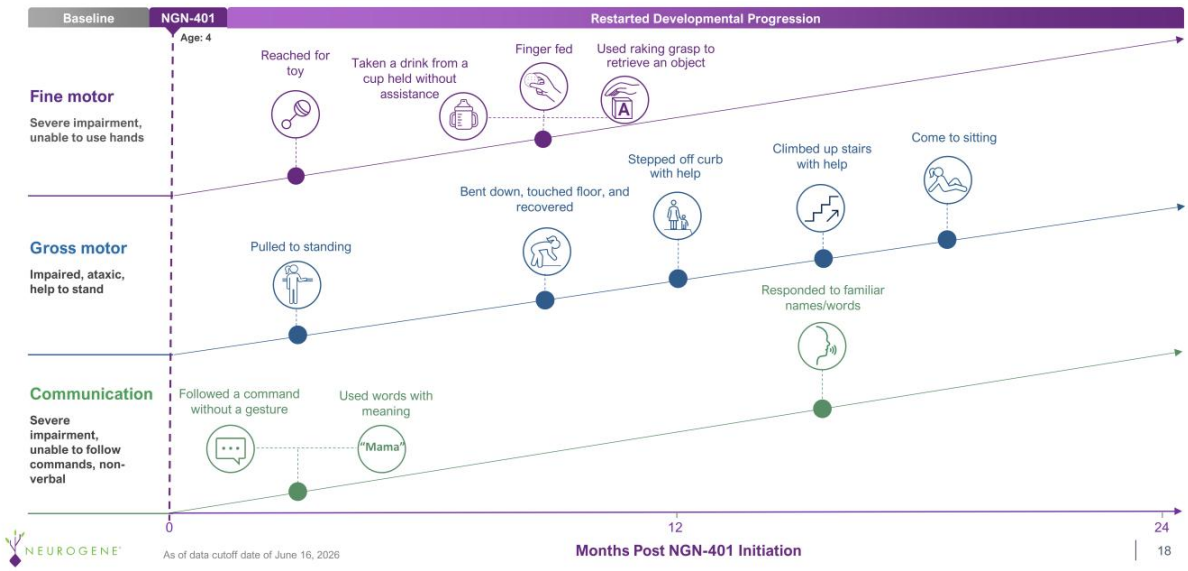
At baseline (Age 7): **Impaired hand use,** raking, no ability to hold objects | **Gross motor impairment,** ataxic, unable to climb stairs | **Severe communication impairment,** unable to follow commands

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

As of data cutoff date of June 16, 2026

Pt:2 Developmental Milestones Gained in Ordered, Developmental Progression



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

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

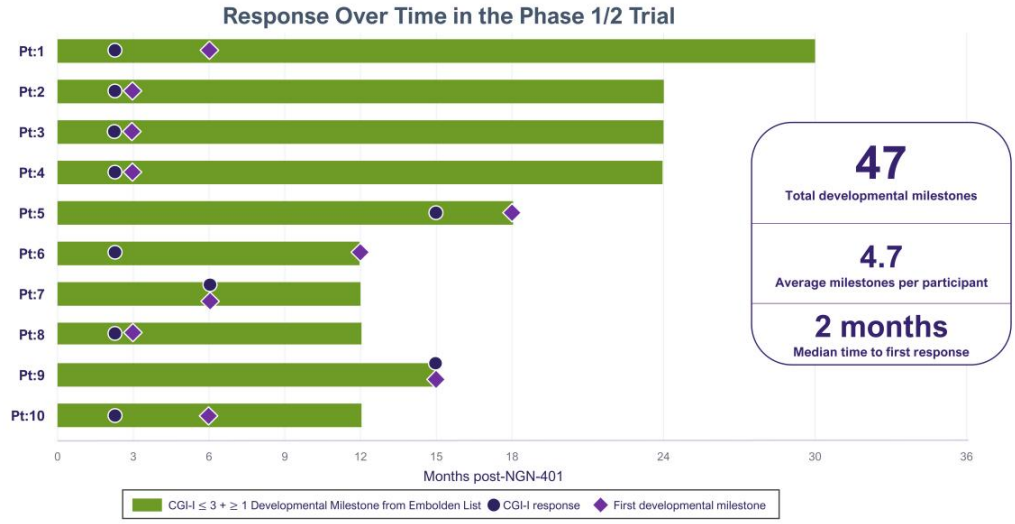
Pt:4 Multidomain Improvements Led to New Abilities Beyond Developmental Milestones

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

Rapid Response Observed Post NGN-401 that Deepened Over Time

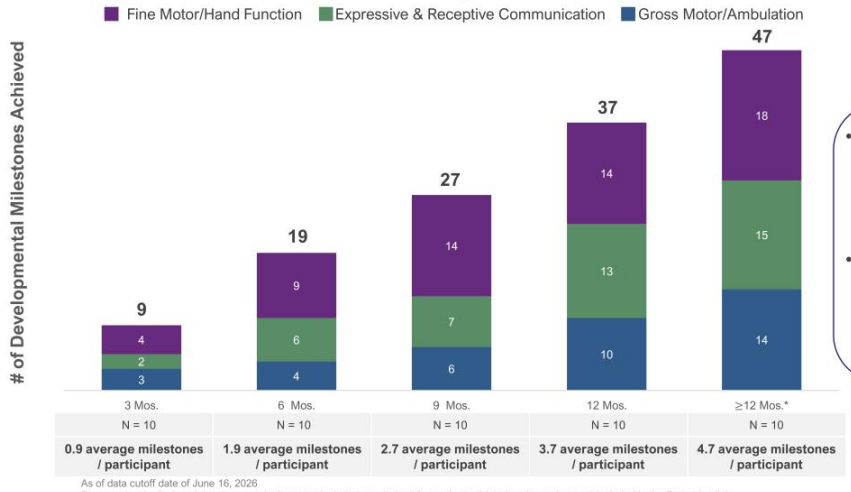


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As of data cutoff date of June 16, 2026
Four previously disclosed developmental milestones included are derived from other validated scales and are not included in the Embolden list:
Pt. 1 - Heel to toe walking – 9 mos.; Pt. 2 – Bent down, touched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

Developmental Milestones Increased by Domain Over Time Post-NGN-401 Treatment



3 Mos.	6 Mos.	9 Mos.	12 Mos.	≥12 Mos.*
N = 10	N = 10	N = 10	N = 10	N = 10
0.9 average milestones / participant	**1.9 average milestones / participant**	**2.7 average milestones / participant**	**3.7 average milestones / participant**	**4.7 average milestones / participant**

- **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

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

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

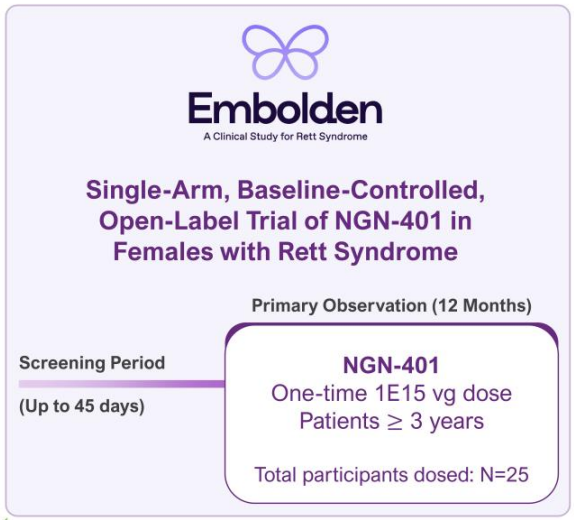
NGN-401 Phase 1/2 Data (N=10)		
Developmental Milestones	% of participants gained ≥1 developmental milestone at ≥12 months	**100%**
	Average milestones gained per participant	**4.7**
	Total participants achieving: - ≥2 milestones - Response on ≥2 domains	**7 out of 10**
	Average # milestones at: - 6 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

Dosing Completed in Embolden Registrational Trial to Support Planned BLA Submission



Primary Endpoint at 12 Months

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

9 of 25 participants, or 36% response rate, 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

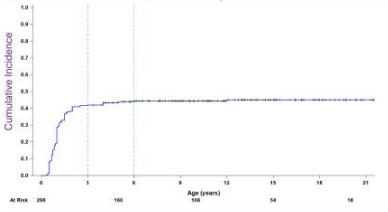
Natural History Study Cumulative Incidence Model Shows Beyond Age 3, Milestone Gains Are Rare with Minimal Difference at Age 6

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

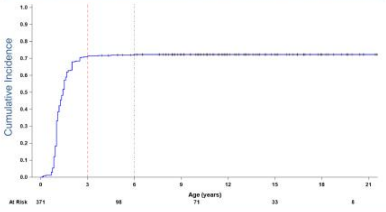
% ever gained/regained: **46%** | At ≥3 years: **4.2%** | At ≥6 years: **1.3%**



Gross Motor

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

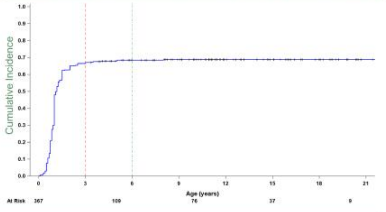
% ever gained/regained: **72%** | At ≥3 years: **1.5%** | At ≥6 years: **0.3%**



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%**

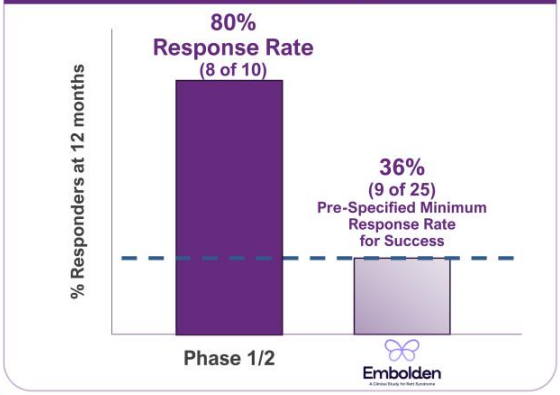
Ex: Participant **Age 14**
2 milestones gained
Age-matched RNHS analysis cumulative incidence rate: **0.0%**

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IRSF Scientific Meeting 2026
RNHS: Rett Syndrome Natural History Study

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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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 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

- Completion of PPQ runs by year-end 2026
- Topline Data from Embolden in 2H 2027
- BLA Submission for NGN-401
- Continue Additional Commercial-Readiness Activities

Strong Cash Balance into 1Q 29

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

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APPENDIX: ADDITIONAL NGN-401 DATA

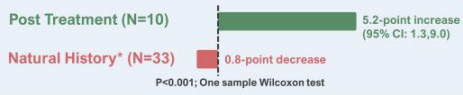
RSGMS Phase 1/2 Data Showed Meaningful Gains in Gross Motor Function

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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*Bisgaard AM *et al. Front Neurol* 2026; 16: 1702703
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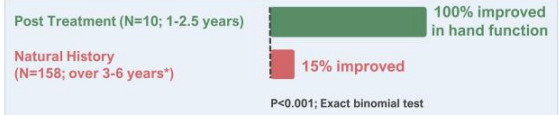
RSHFS Phase 1/2 Data Showed Meaningful Gains in Hand Function

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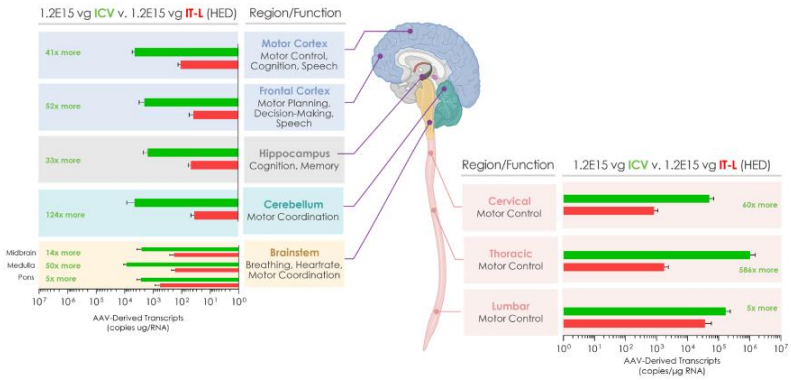
*Downs J *et al. J Pediatr* 2021; 237: 244–249; PMID: 34214590

IRSF Scientific Meeting 2026

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

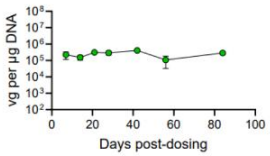
An estimated 30,000 ICV procedures are performed by neurosurgeons annually in the U.S. and require minimal downtime/recovery



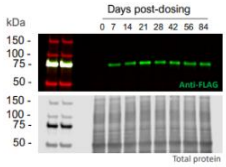
ICV = Intracerebroventricular
IT-L = intrathecal lumbar
ESGCT 2025

Rapid and Durable Expression of Full-Length *MECP2* Following ssAAV Delivery in Preclinical Study, Consistent with Early Phase 1/2 Clinical Benefit

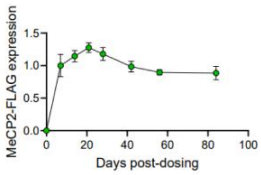
ssAAV is required to accommodate full-length *MECP2* gene; ssAAV-mediated MeCP2 expression detectable by day 7 post dosing and sustained for the 3-month duration of the study



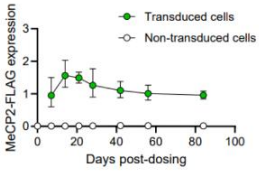
Quantification of vg in neocortex



Immunoblot of anti-FLAG expression in neocortex



Quantification of immunoblot data normalized to Day 7*



Single nuclei flow cytometry analysis of neocortical neurons normalized to Day 7*

ESGCT 2026
ssAAV: single-stranded AAV
*Datapoints in plots represent mean ± SEM, N=3-5