NEUROGENE®

EVERY BREAKTHROUGH BEGINS WITH BELIEF

Corporate Presentation

January 2026

Disclaimer

Forward Looking Statements

This communication contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. These statements may discuss goals, intentions and expectations as to future plans, trends, events, results of operations or financial condition, or otherwise, based on current expectations and beliefs of the management of Neurogene, as well as assumptions made by, and information currently available to, management of Neurogene, including, but not limited to, statements regarding: the therapeutic potential and utility, efficacy and clinical benefits of its programs, including its EXACT™ technology and NGN-401; the potential for commercial approval of NGN-401 and the speed with which any such approval might be obtained; market opportunities for Neurogene's product candidates, including the estimated prevalence of Rett syndrome and expected levels of demand for NGN-401; the safety and tolerability profile of NGN-401; the applicability of reported interim results from the NGN-401 Phase 1/2 clinical trial to other participants or potential participants, including adolescent or adult participants; any extrapolation of interim trial results on the likelihood of gaining approval of NGN-401 from the FDA or any other regulator; trial designs and clinical development plans for the Embolden™ registrational clinical trial of NGN-401 for Rett Syndrome, including timing of anticipated enrollment and completion of the enrollment in this clinical trial; the response rate, expected durability and deepening of clinical data results from the NGN-401 clinical trials; expected timing for release of additional data from the Phase 1/2 clinical trial of NGN-401; the potential superiority of ICV administration and delivery of a full MECP2 gene as against other potential gene therapies; the potential for NGN-401 to be a best-in-class or first-in-class gene therapy for Rett syndrome; patient, caregiver and KOL sentiments relating to priorities on selecting potential gene therapy treatments; expectations related to payer reimbursement for NGN-401 if approved, including estimates related to potential reimbursement rates, the ease of obtaining reimbursement and the sentiments of payors with respect to value placed on certain outcomes and any impact of potentially implementing an outpatient regimen for NGN-401; the speed with which Neurogene could convert existing clinical trial sites to commercial sites if it is successful in obtaining regulatory approval for the commercialization of NGN-401; the potential for success of the Embolden registrational clinical trial of NGN-401 for Rett Syndrome; expected future interactions with or positions of the FDA or foreign regulatory authorities, including the timing and outcome of any such interaction and anticipated benefits of any regulatory designation for Neurogene's product candidates, including the FDA's RMAT designation, the EMA's PRIME designation and participation in the FDA's START program with respect to NGN-401; the benefits of Neurogene's in-house manufacturing capabilities; anticipated early-stage discovery and expectations regarding the initiation of future clinical trials for programs in development; the timing and achievement of any catalyst for value creation for Neurogene; and Neurogene's cash runway, including the time period over which existing cash resources may be sufficient to fund the Company's operations. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as "may," "will," "should," "would," "expect," "anticipate," "plan," "likely," "believe," "estimate," "project," "intend," and other similar expressions or the negative or plural of these words, or other similar expressions that are predictions or indicate future events or prospects, although not all forward-looking statements contain these words. Statements that are not historical facts are forward-looking statements. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: uncertainties regarding interactions with and feedback received from FDA staff regarding the Embolden registrational trial including the risk that the FDA could change its requirements for the Embolden trial; the ability to raise additional capital to finance operations; the ability of Neurogene to report its data on the predicted timeline; the ability of Neurogene to effectively use the RMAT designation, PRIME designation or START program to accelerate development of NGN-401; the potential for negative impacts to patients dosed in the ongoing clinical trials for NGN-401; the ability to advance product candidates through non-clinical and clinical development; the ability to obtain regulatory approval for, and ultimately commercialize, Neurogene's product candidates; Neurogene's limited experience in designing and conducting clinical trials; the ability to identify and pivot to other programs, product candidates that may be more profitable or successful than Neurogene's current product candidates; expectations regarding the potential tolerability, safety or efficacy for NGN-401; the ability to attract, hire, and retain skilled executive officers and employees; reliance on third parties, contract manufacturers, and contract research organizations; the ability of Neurogene to protect its intellectual property and proprietary technologies; risks related to Neurogene's ability to correctly estimate its operating expenses, including its projected cash runway; and legislative, regulatory, political and economic developments and general market conditions.

The foregoing review of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors included in the Company's most recent Annual Report on Form 10-K and Quarterly Reports on Form 10-Q filed with the Securities and Exchange Commission, as well as risk factors associated with companies, such as Neurogene, that operate in the biopharma industry. These forward-looking statements involve a number of risks, uncertainties (some of which are beyond Neurogene's control) or other assumptions that may cause actual results or performance to be materially different from those expressed or implied by these forward-looking statements. Nothing in this communication should be regarded as a representation by any person that the forward-looking statements set forth herein will be achieved or that the contemplated results of any such forward-looking statements will be achieved. Forward-looking statements in this communication speak only as of the day they are made and are qualified in their entirety by reference to the cautionary statements herein. Except as required by applicable law, Neurogene undertakes no obligation to revise or update any forward-looking statement, or to make any other forward-looking statements, whether as a result of new information, future events or otherwise.

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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.

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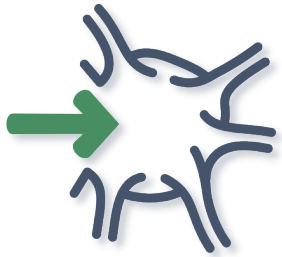
OUR MISSION

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

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

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; multiple participants dosed in 4Q'25

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

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

Upcoming Milestones

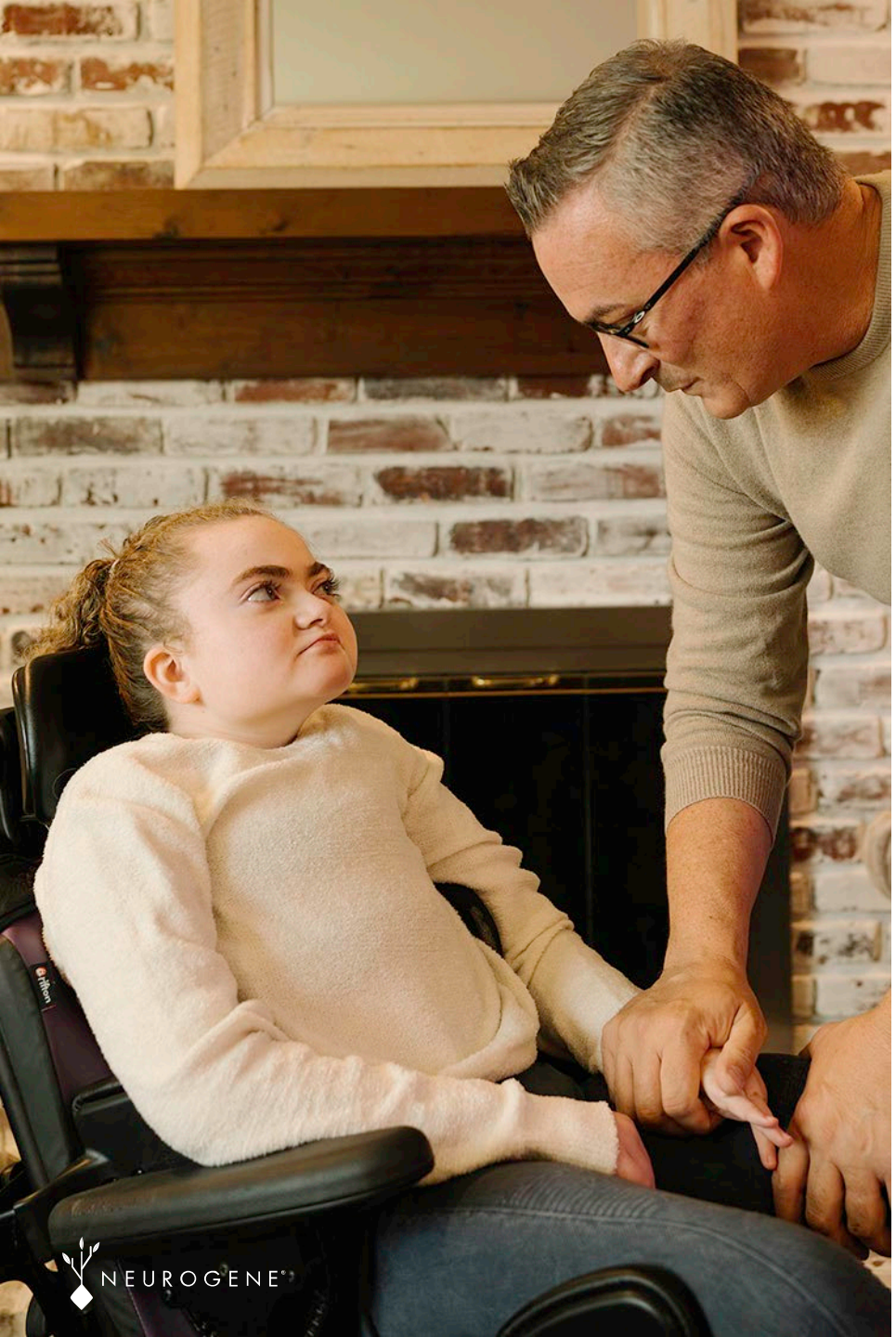
- Complete dosing of Embolden **expected in 2Q'26**
- Presentation of 12+ months Phase 1/2 data for all 10 participants **planned for mid-2026**

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

2025 Accomplishments Set Up for Pivotal Year in 2026

NGN-401 for Rett Syndrome

- Completed enrollment of Phase 1/2 trial
- Obtained FDA alignment on Embolden registrational trial
- Dosed multiple participants in Embolden
- Reported positive interim Phase 1/2 data
- Received PRIME designation from EMA, the fifth special regulatory designation



Rett Syndrome: Rare, Debilitating, Progressive, Neurodevelopmental Disorder

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

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

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

Hallmark features:

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

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

Rett Syndrome is One of the More Prevalent Rare Diseases

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

1:10,000 females
Worldwide incidence[2]

Clear Unmet Need

No disease-modifying treatment available

Only treatment options
are limited to symptom management

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

Payors are Receptive to Reimbursement[3]

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

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

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

NEUROGENE®

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®

ICV = Intracerebroventricular

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NGN-401 PHASE 1/2 TRIAL

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

Trial evaluating 1E15 vg dose of NGN-401

n=8	n=2
Ages 4-10	***Ages ≥ 11***

Trial Overview

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

Key Eligibility Criteria:

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

Baseline Characteristics of the Pediatric Participants in Phase 1/2

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

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

As of data cutoff date of October 30, 2025

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

Key Findings from Interim Phase 1/2 Data

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

As of data cutoff date of October 30, 2025; Neurogene press release (November 12, 2025)

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

Caregivers Consistently Highlight Improvements in Core Domains are the Priority

Fine Motor/Hand Function Improvements

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

Expressive & Receptive Communication Improvements

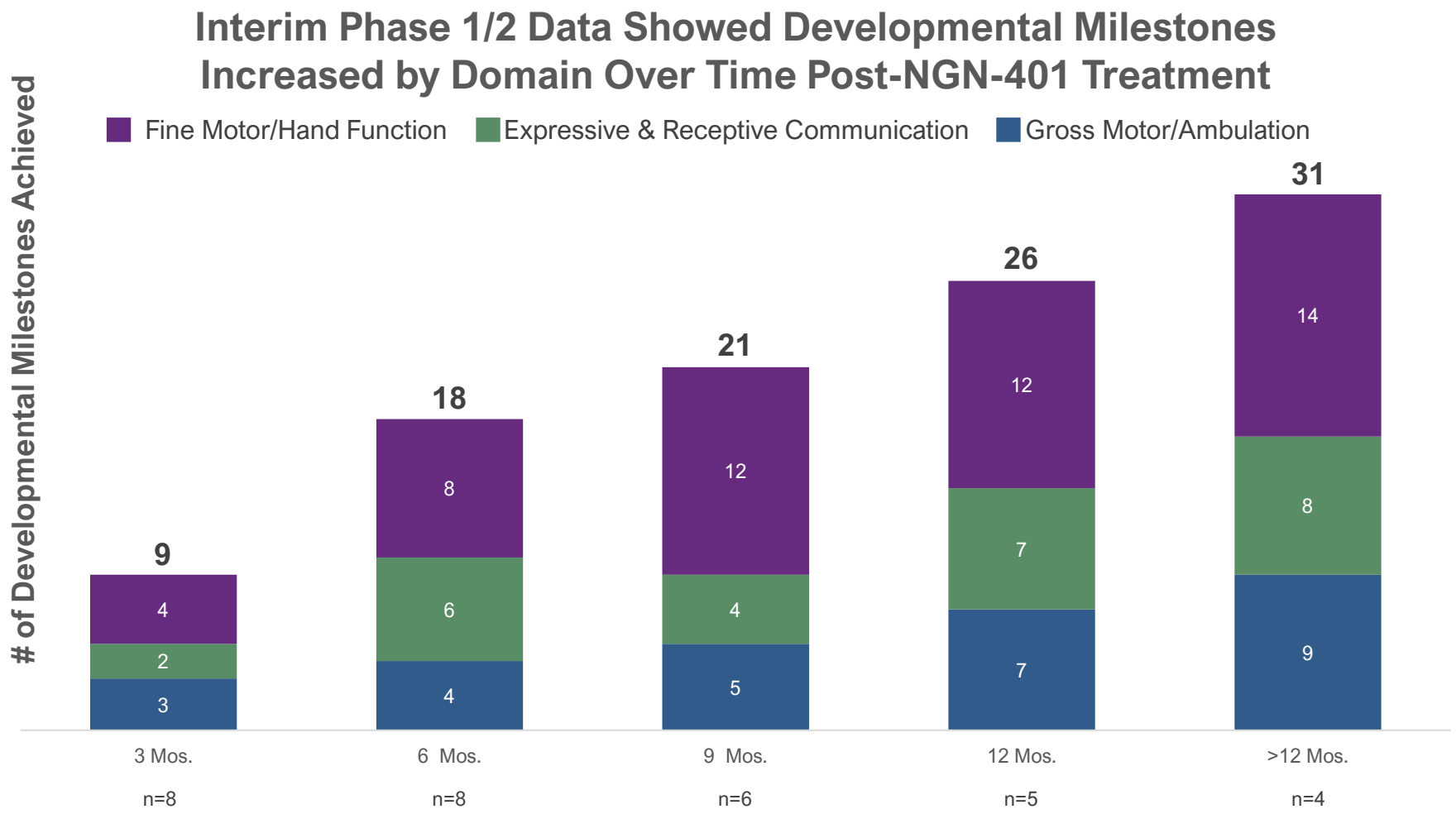
- Expressing needs, following instructions simplifying daily routines and strengthening connections
- E.g., Making choices, using words with meaning

Gross Motor/Ambulation Improvements

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

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

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



As of data cutoff date of October 30, 2025

Embolden
A Clinical Study for Rett Syndrome

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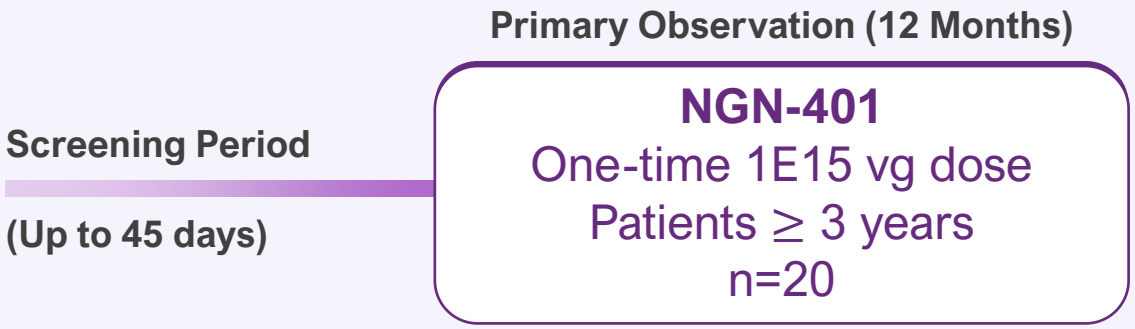
EMBOLDEN™ REGISTRATIONAL TRIAL OVERVIEW

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

Embolden
A Clinical Study for Rett Syndrome

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



Primary Endpoint at 12 Months

Responder-based composite endpoint defined as:

- **CGI-I of ≤ 3** and
- **Gain from baseline of any one developmental milestone**

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

Key Secondary Endpoints

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

Developmental Milestones

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

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

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

Embolden
A Clinical Study for Rett Syndrome

Fine Motor/ Hand Function

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

Gross Motor/Ambulation

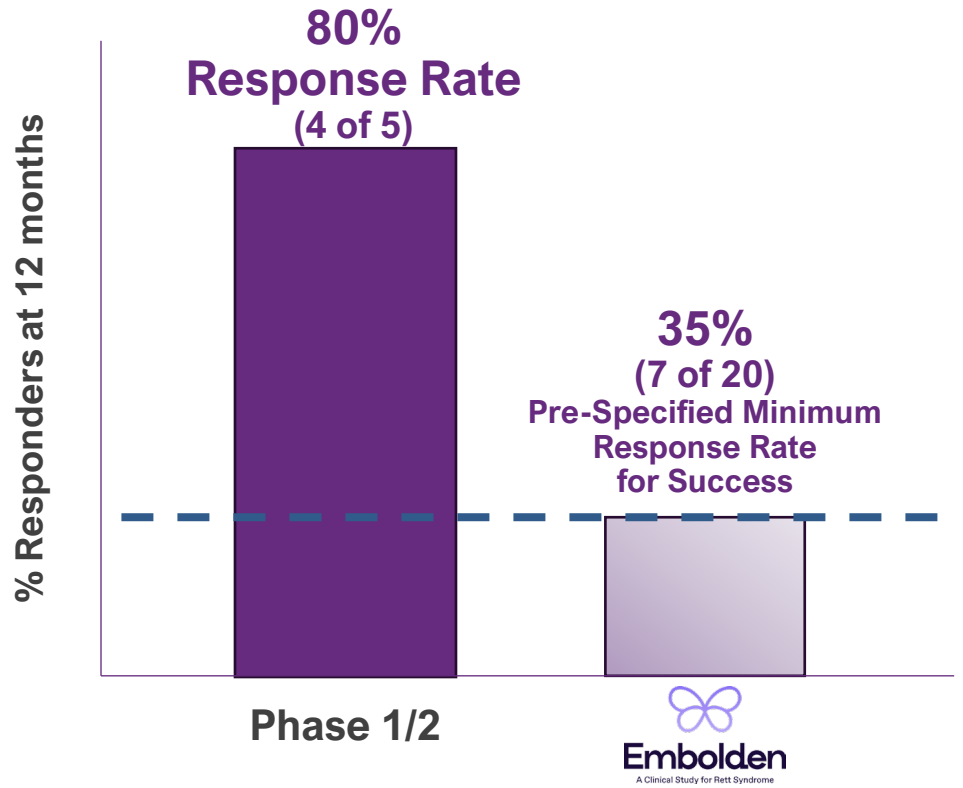
- Sat with support when placed
- Sat without support when placed
- Come to sitting
- Pulled to standing
- Stood while holding on
- Stood independently
- Cruised around furniture or holding on to someone
- Walked independently
- Climbed up stairs with help
- Climbed up stairs without help
- Climbed down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

Communication

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

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

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



Key Design Elements Carried Into Embolden

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

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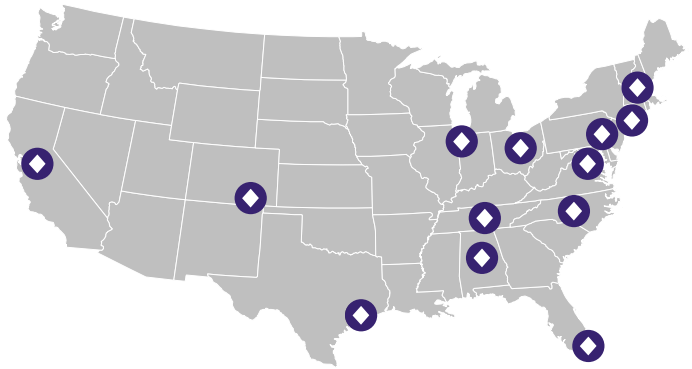
As of data cutoff date of October 30, 2025

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

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

TODAY

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

ANTICIPATED 2026 MILESTONES

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

Strong Cash Balance (1Q'28)

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

APPENDIX:
INTERIM PHASE 1/2 DATA UPDATE
PARTICIPANT VIGNETTES AND SAFETY OVERVIEW

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

Pt:1 Baseline: 7 Yrs Old

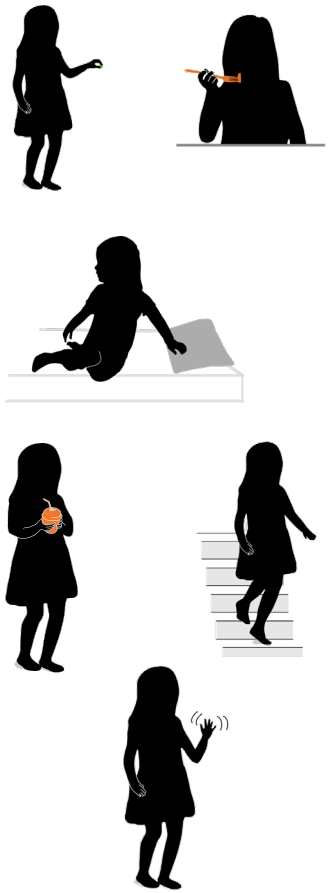
Raking, no ability to hold objects

Walking, ataxic gait, no ability to climb stairs

Severe impairment, unable to follow commands, indicate wishes

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

Post Treatment with NGN-401



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

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

Pt:2 Baseline: 4 Yrs Old

Severe impairment, unable to use hands

Impaired, ataxic, help to stand

Severe impairment, unable to follow commands, non-verbal

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

Post Treatment with NGN-401

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

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

Pt:3 Baseline: 6 Yrs Old

Severe Dysphagia

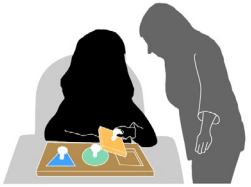
Raking grasp

Cannot sit, stand or walk independently

Cannot follow commands

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

Post Treatment with NGN-401



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

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

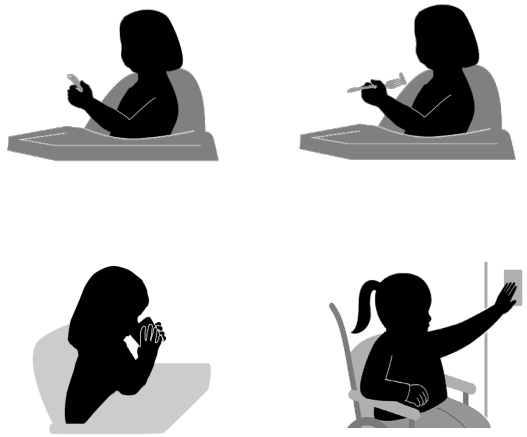
Pt:4 Baseline: 7 Yrs Old

Raking grasp, unable to hold objects

Unable to follow commands

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

Post Treatment with NGN-401



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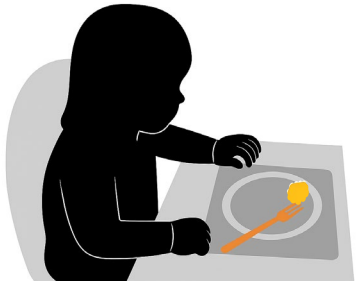
As of data cutoff date of October 30, 2025
Images are representative of developmental milestones

Pt:7 Gained 1 Developmental Milestone at Early Timepoint

Pt:7 Baseline:
6 Yrs Old



Could not use utensils

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

Post Treatment with NGN-401

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

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

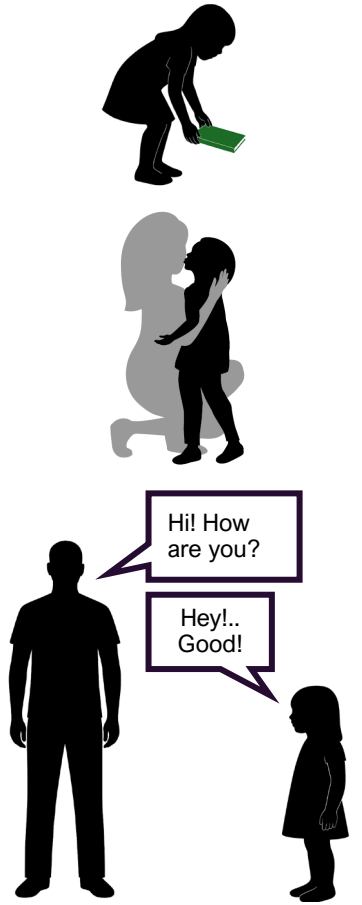
Pt:8 Baseline: 8 Yrs Old

Unable to bend at waist

Unable to follow commands, cannot use words with meaning

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

Post Treatment with NGN-401



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

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

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

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

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