



EVERY BREAKTHROUGH BEGINS WITH BELIEF

Corporate Presentation
March 2026



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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 commercial approval of NGN-401 and the speed with which any such approval might be obtained; market opportunities for Neurogene's product candidates, including the estimated prevalence of Rett syndrome and expected levels of demand for NGN-401; the safety and tolerability profile of NGN-401; the applicability of reported interim results from the NGN-401 Phase 1/2 clinical trial to other participants or potential participants, including adolescent or adult participants; any extrapolation of interim trial results on the likelihood of gaining approval of NGN-401 from the FDA or any other regulator; trial designs and clinical development plans for the Embolden™ registrational clinical trial of NGN-401 for Rett Syndrome, including timing of anticipated enrollment and completion of the enrollment in this clinical trial and the timing and the expected timeline of our Process Performance Qualification (PPQ) activities for our chemistry, manufacturing and controls (CMC) requirements; the response rate, expected durability and deepening of clinical data results from the NGN-401 clinical trials; expected timing for release of additional data from the Phase 1/2 clinical trial of NGN-401; the potential superiority of ICV administration and delivery of a full MECP2 gene as against other potential gene therapies; the potential for NGN-401 to be a best-in-class or first-in-class gene therapy for Rett syndrome; patient, caregiver and KOL sentiments relating to priorities on selecting potential gene therapy treatments; expectations related to payer reimbursement for NGN-401 if approved, including estimates related to potential reimbursement rates, the ease of obtaining reimbursement and the sentiments of payers with respect to value placed on certain outcomes and any impact of potentially implementing an outpatient regimen for NGN-401; the ability to and speed with which Neurogene could convert existing clinical trial sites to commercial sites if it is successful in obtaining regulatory approval for the commercialization of NGN-401; the potential for success of the Embolden registrational clinical trial of NGN-401 for Rett Syndrome; expected future interactions with or positions of the FDA or foreign regulatory authorities, including the timing and outcome of any such interaction and anticipated benefits of any regulatory designation for Neurogene's product candidates, including the FDA's Breakthrough Therapy designation and RMAT designation, the EMA's PRIME designation and participation in the FDA's START program with respect to NGN-401; the benefits of Neurogene's in-house manufacturing capabilities; anticipated early-stage discovery and expectations regarding the initiation of future clinical trials for programs in development; the timing and achievement of any catalyst for value creation for Neurogene; and Neurogene's cash runway, including the time period over which existing cash resources may be sufficient to fund the Company's operations. 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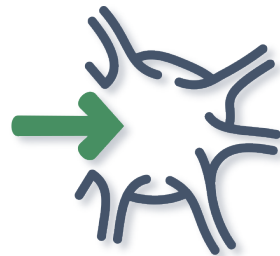
OUR MISSION

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
- 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
- 100% of participants enrolled in Embolden and >50% of participants dosed

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

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

Upcoming Milestones

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

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

NGN-401 for Rett Syndrome





Rett Syndrome: Rare, Debilitating, Progressive, Neurodevelopmental Disorder

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

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

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

Hallmark features:

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

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

Rett Syndrome is One of the More Prevalent Rare Diseases



~15,000 – 20,000 patients

Major market prevalence
US, EU and UK¹

1:10,000 females

Worldwide incidence²

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 Reimbursement³

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



Payors are familiar with Rett syndrome

Decades-long survival supports premium-priced durable gene therapy

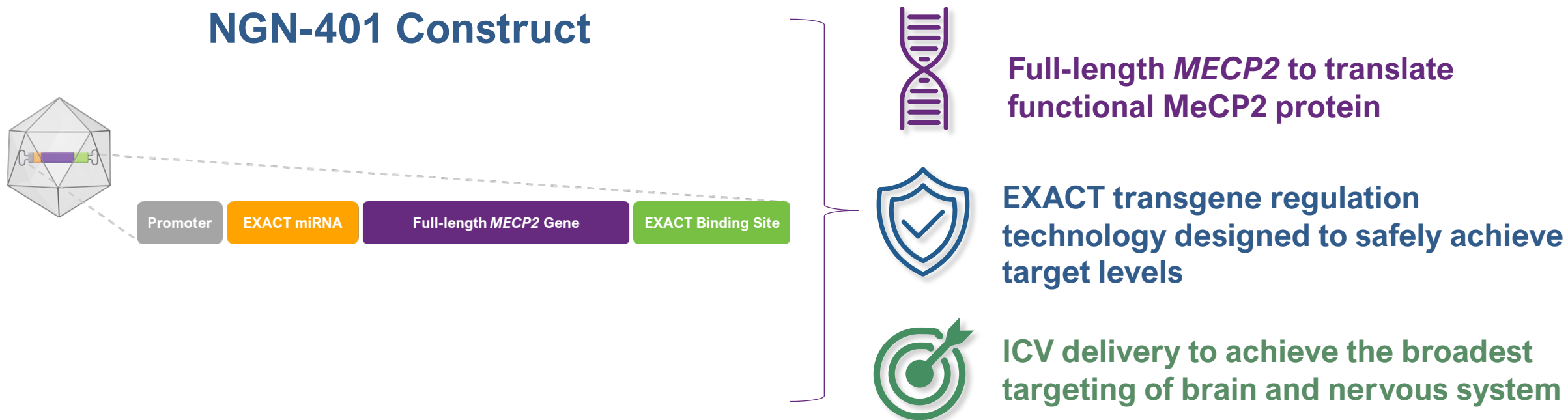
¹ Major market prevalence based on internal estimates; U.S. prevalence estimate based on published incidence rates; Laurvick CL, et al. J Pediatr 2006;148(3):347–35.

² WW incidence estimate based on published incidence rates; Pini G, et al. Orphanet Journal of Rare Diseases (2016) 11:132.

³ Internal market research

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

NGN-401 Construct

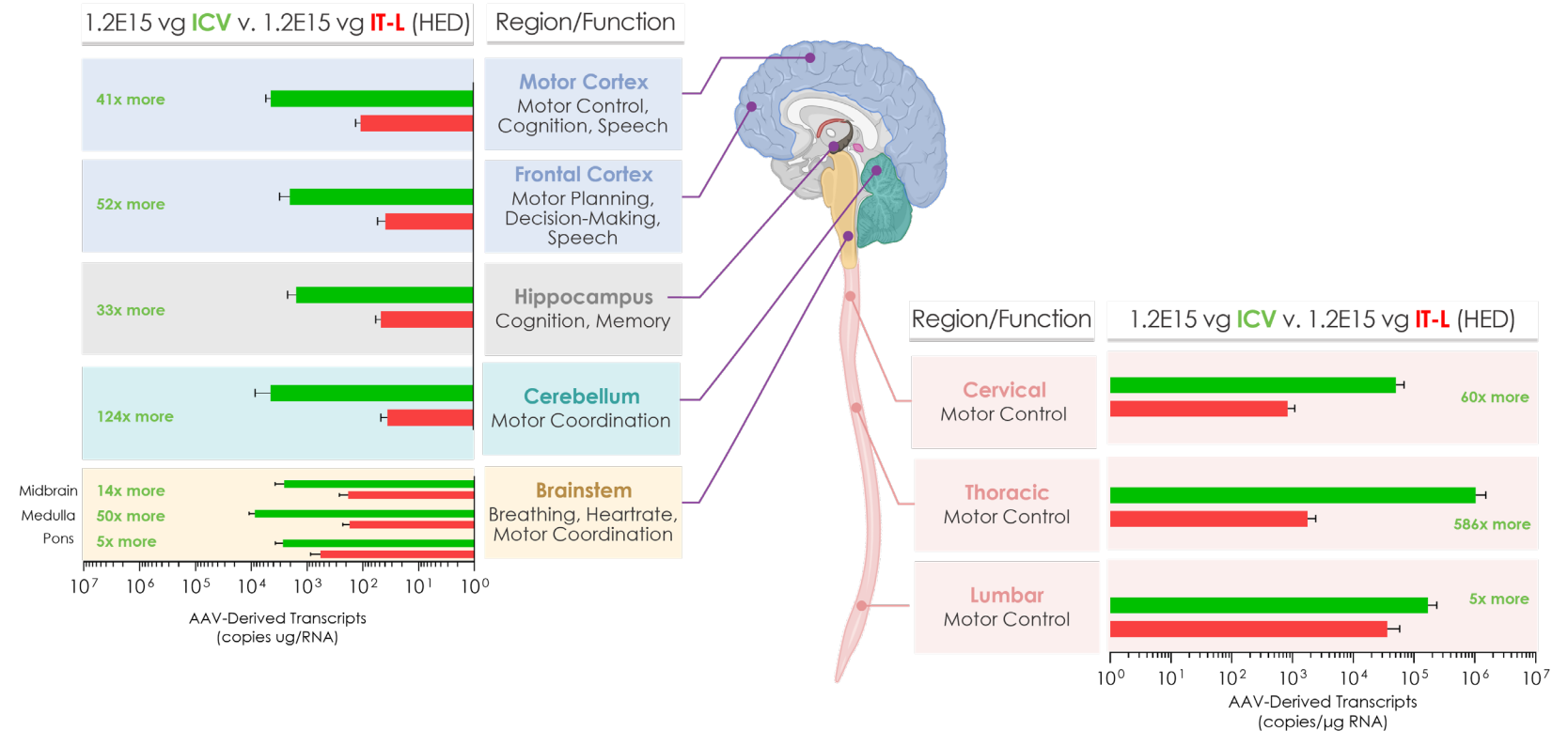


Only Gene Therapy Designed to Safely Deliver Full-length Protein to Key Areas of the Brain and Nervous System to Maximize Benefit

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

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

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



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
Ages 4-10

n=2
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 III	5 Markedly III	5 Markedly III	5 Markedly III	5 Markedly III	5 Markedly III	4 Moderately III	4 Moderately III
Genetic Variant Severity	Mild	Severe	Severe	Severe	Severe	Moderate	Mild-Moderate	Mild-Moderate
Time Post-Dosing (Months)	24	18	18	15	12	6	6	6

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

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

Key Findings from Interim Phase 1/2 Data

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

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

Caregivers Consistently Highlight Improvements in Core Domains are the Priority



Fine Motor/Hand Function Improvements

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



Expressive & Receptive Communication Improvements

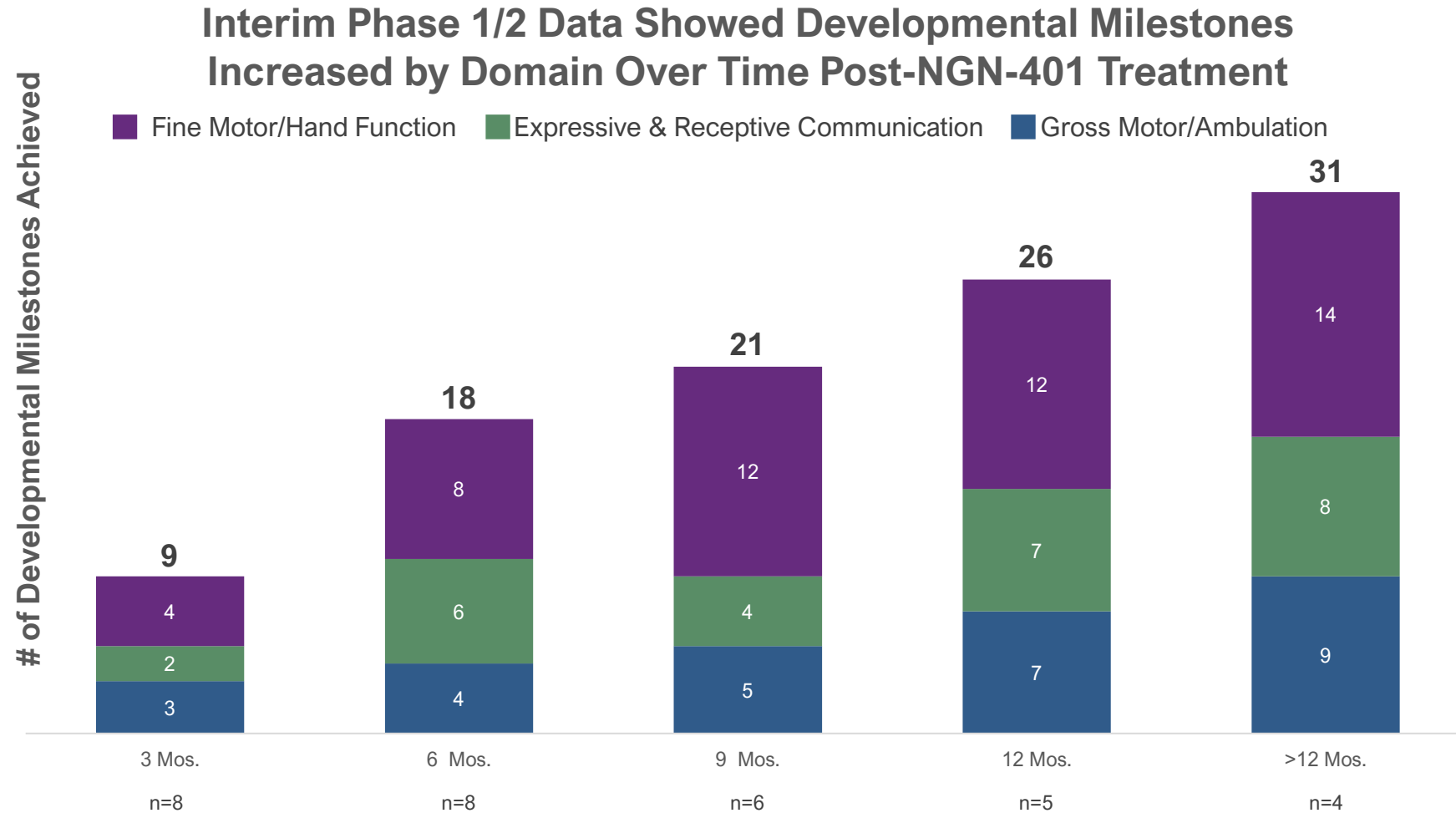
- ◆ Expressing needs, following instructions simplifying daily routines and strengthening connections
- ◆ E.g., Making choices, using words with meaning



Gross Motor/Ambulation Improvements

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

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




















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

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

Improvements in Key Domains Lead to:

- Multidomain improvements**
- Participation in activities of daily living**
- Improvements in health-related quality of life**



Fine Motor/Hand Function	Gross Motor/Ambulation	Communication	Autonomic
<p>Less dependence on caregivers for basic needs</p>	<p>Reduced physical burden for caregivers for activities of daily living</p>	<p>Can communicate needs & wants, and foster greater social connections</p>	<p>Enhanced comfort and reduced distress</p>
<p> Uses hands to self-feed, drink, play with toys, self-soothe</p>	<p> Sits without support</p>	<p> Says words with meaning</p>	<p> Improvements in constipation</p>
<p> Decreased to no stereotypies</p>	<p> Walks up and down stairs unassisted</p>	<p> Follows daily routines, e.g., getting ready for school</p>	<p> Drinks clear liquids, eats solids for first time</p>
<p> Uses utensils to self-feed</p>	<p> Bends at waist and stands back up</p>	<p> Communicates needs and wants; can ID colors</p>	<p> Absent aspiration pneumonia events</p>
	<p> Bears more weight for ease of transfer</p>	<p> Engages with friends and family; shows affection</p>	<p> Sleeps through the night</p>
	<p> Can get on/off furniture, out of bathtub, into car unassisted</p>	<p> Starting/ improved communication with use of assisted device</p>	

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

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

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

“The other day she...**ran a couple steps** while out...”

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

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

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

“She is **happier.**”

“**I am grateful** [NGN-401] has seemed to **benefit [her] health and life...**”

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

“**She is so happy all the time and calm.**”

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

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

“**She kissed me for the first time ever.**”



NGN-401 Remains Generally Well Tolerated at the 1E15 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



EMBOLDEN™

REGISTRATIONAL TRIAL OVERVIEW



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



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

Primary Observation (12 Months)

Screening Period
(Up to 45 days)

NGN-401
One-time 1E15 vg dose
Patients \geq 3 years
n=20

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

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

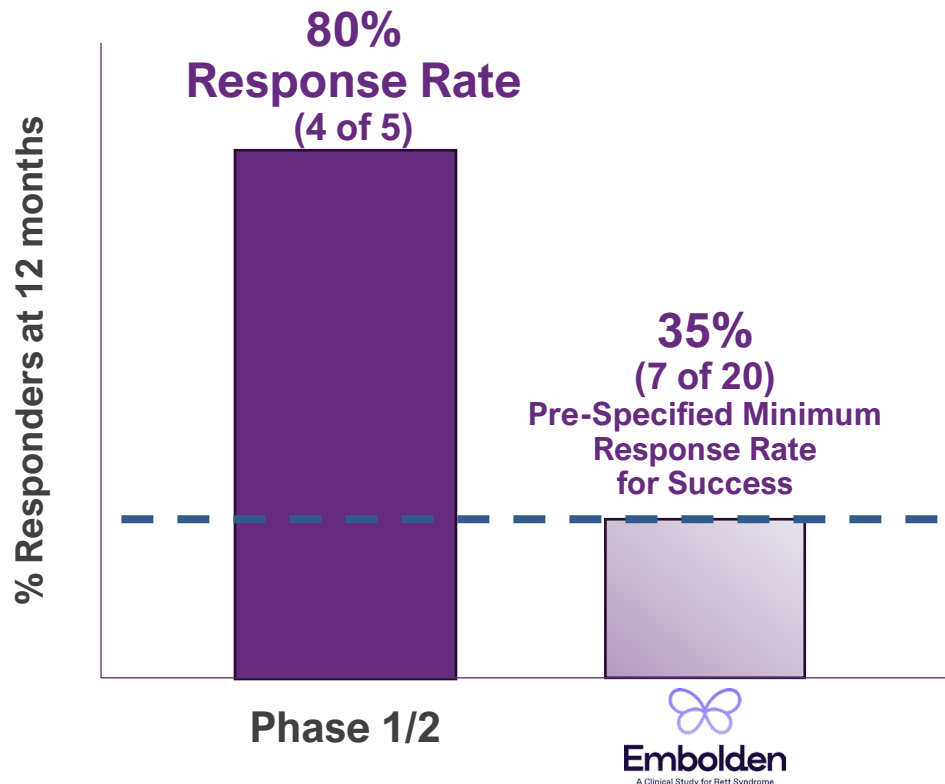


Communication

- ◆ 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

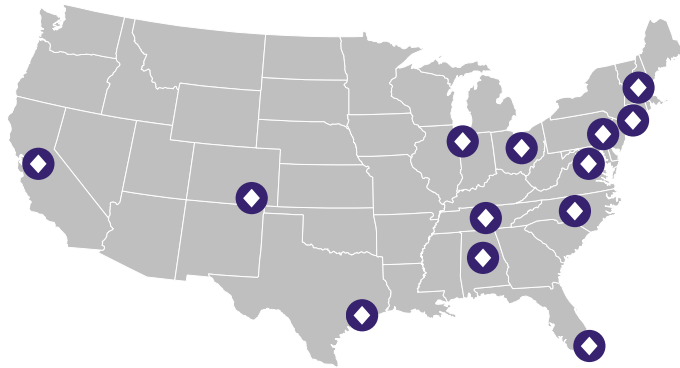
COMMERCIAL READINESS & KEY ANTICIPATED MILESTONES



Building a Strong Foundation for Commercialization



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



◆ Embolden Clinical Trial Sites at Rett Centers of Excellence



Internal CMC capabilities and manufacturing facility to produce commercial product



Payor research confirms strong reimbursement potential for NGN-401



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



Outpatient pathway optionality intended to further simplify reimbursement

Wholly Owned and Fully Integrated In-House AAV Manufacturing



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



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

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

2025



ANTICIPATED 2026 MILESTONES



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



Complete Dosing in Embolden Registrational Trial

Expected Timing: 2Q'26



Present Phase 1/2 Data:

12+ month data for all 10 participants
Expected Timing: Mid-2026



Start PPQ campaign

Expected Timing: Mid-2026



Initiation of Additional Commercial-Readiness Activities



Strong Cash Balance (1Q'28)

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

APPENDIX: INTERIM PHASE 1/2 DATA UPDATE PARTICIPANT VIGNETTES



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

**Pt:1 Baseline:
7 Yrs Old**

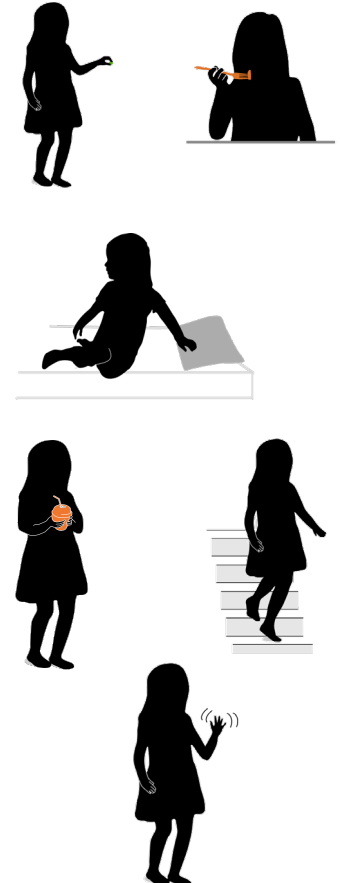
Raking, no ability
to hold objects

Walking, ataxic gait, no
ability to climb stairs

Severe impairment,
unable to follow
commands, indicate
wishes

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

**Post Treatment
with NGN-401**



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

Pt:2 Baseline:
4 Yrs Old

Pt:2 Developmental Milestones
Post-NGN-401

Months Post-NGN-401

3 6 9 12 15 18

Severe impairment,
unable to use hands

Fine Motor/Hand Function

Reached for toy	✓	✓	✓	✓	✓	✓
Used raking grasp to retrieve an object			✓	✓	✓	✓
Finger fed			✓	✓	✓	✓
Taken a drink from a cup held without assistance			✓	✓	✓	✓

Impaired, ataxic,
help to stand

Gross Motor/Ambulation

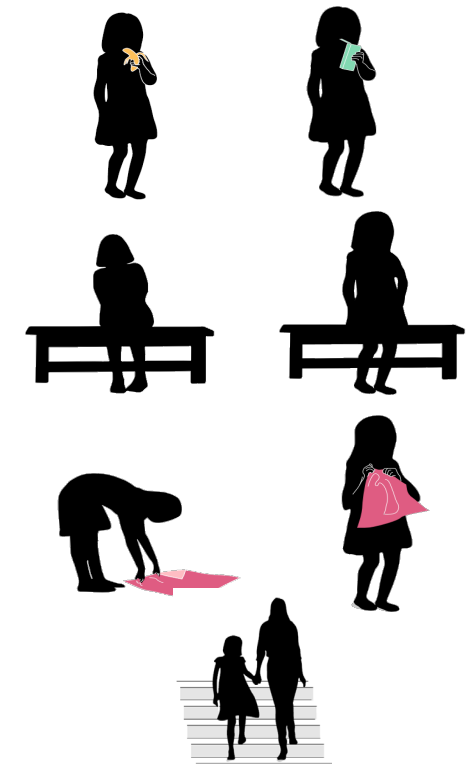
Pulled to standing	✓	✓	✓	✓	✓	✓
Bent down, touched floor, and recovered*			✓	✓	✓	✓
Stepped off curb with help*				✓	✓	✓
Climbed up stairs with help					✓	✓

Severe impairment,
unable to follow
commands, non-verbal

Communication

Followed a command without a gesture	✓	✓	✓	✓	✓	✓
Used words with meaning	✓	✓	✓	✓	✓	✓

Post Treatment
with NGN-401



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

Pt:3 Baseline:
6 Yrs Old

Pt:3 Developmental Milestones
Post-NGN-401

Months Post-NGN-401

3 6 9 12 15 18

Raking grasp

Fine Motor/Hand Function

Used a pincer grasp (either refined or modified)

✓ ✓ ✓ ✓ ✓

Finger fed

✓ ✓ ✓ ✓

Transferred an object from one hand to the other

✓ ✓ ✓ ✓

Cannot sit, stand or
walk independently

Gross Motor/ Ambulation

Sat without support when placed

✓ ✓ ✓ ✓ ✓ ✓

Cannot follow
commands

Communication

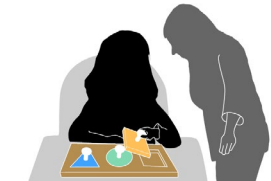
Followed command without gesture

✓ ✓ ✓

Followed command with gesture

✓

Post Treatment
with NGN-401



Severe
Dysphagia

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

Pt:4 Baseline:
7 Yrs Old

Pt:4 Developmental Milestones
Post-NGN-401

Months Post-NGN-401

3 6 9 12 15

Raking grasp, unable
to hold objects

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

✓ ✓ ✓ ✓ ✓

Unable to follow
commands

Communication

Followed command without a gesture

✓ ✓ ✓ ✓

Post Treatment
with NGN-401



Pt:7 Gained 1 Developmental Milestone at Early Timepoint

Pt:7 Baseline:
6 Yrs Old

Pt:7 Developmental Milestones
Post-NGN-401

Months Post-NGN-401

3

6

Could not use utensils

Fine Motor/Hand Function

Used a spoon/fork to eat without assistance



Post Treatment
with NGN-401



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

Pt:8 Baseline: 8 Yrs Old	Pt:8 Developmental Milestones Post-NGN-401	Months Post-NGN-401	
		3	6
Unable to bend at waist	Gross Motor/ Ambulation		
	Bent down, touched floor, and recovered*	✓	✓
Unable to follow commands, cannot use words with meaning	Communication		
	Followed command with a gesture		✓
	Used words with meaning		✓

