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EVERY BREAKTHROUGH BEGINS WITH BELIEF



Corporate Presentation

November 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 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. 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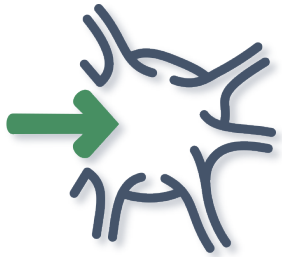
OUR MISSION

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
- 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

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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

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Major market prevalence based on internal estimates; U.S. prevalence estimate based on published incidence rates; Laurvick CL, et al. J Pediatr 2006;148(3):347–352.
WW incidence estimate based on published incidence rates; Pini G, et al. Orphanet Journal of Rare Diseases (2016) 11:132.
Cheng et al., BMC Neurology, 2023 (IQVIA Claims, N=5,940)

NGN-401: 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

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

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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

As of data cutoff date of June 16, 2026

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

Phase 1/2 Trial
1E15 vg

Dosing complete (N=10)
Ages 4-10, ≥11 years old

Embolden Registrational Trial
1E15 vg

Dosing complete (N=25)
Ages ≥3 years old

EMBOLDEN PRIMARY ENDPOINT · RESPONDER REQUIRES BOTH AT 12 MONTHS

CGI-I ≤3 + **Gain from baseline of ≥1 developmental milestone**

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

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

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



Fine Motor/ Hand Function

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



Gross Motor/Ambulation

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



Communication

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

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

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

Baseline Assessment

Milestones confirmed absent at baseline — from medical history, caregiver report and video

Post-Treatment Evidence

Milestones documented in **caregiver and CGI videos**

Evaluation Methods

Gain from baseline scored by **independent central rating** using pre-specified Embolden criteria

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

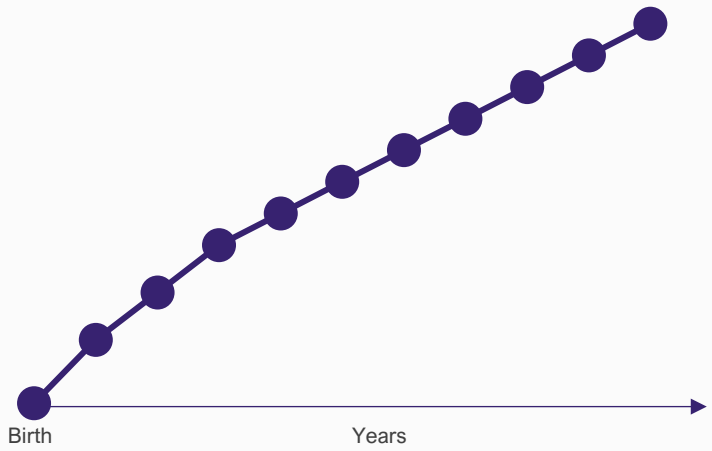
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As of data cutoff date of June 16, 2026
Pt = Participant

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

Typical Development

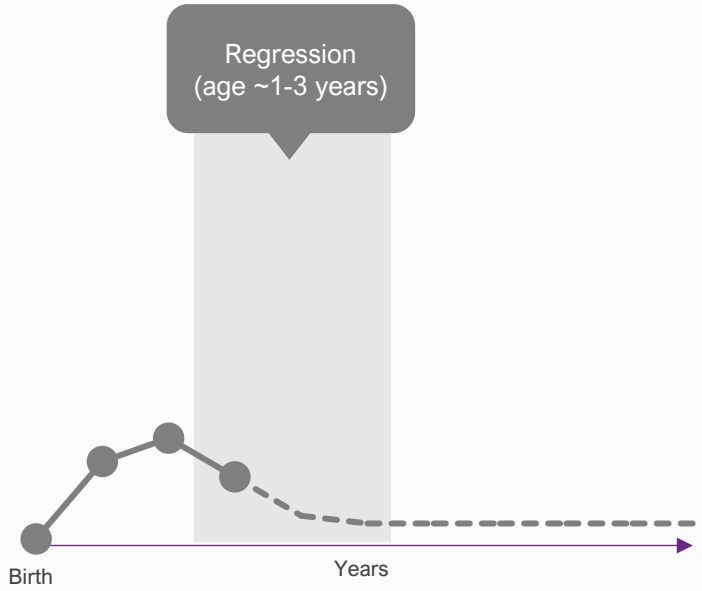
Organized, cumulative acquisition of developmental milestones



Developmental milestones build over time across domains in a coordinated and integrated manner

Rett Syndrome

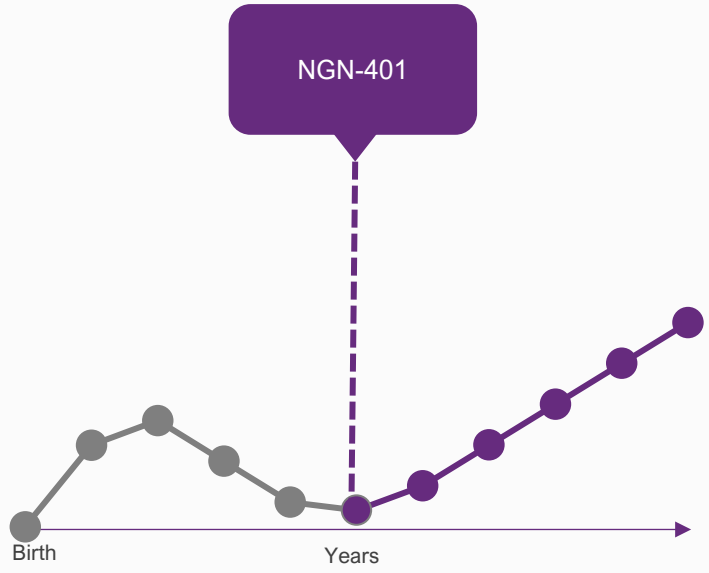
Milestones lost in regression, exceedingly rare to gain milestones post-regression



Simple developmental milestones are gained and then lost during regression, leading to 24/7, lifelong care

Restarted Developmental Progression

NGN-401 has potential to restart the typical developmental progression

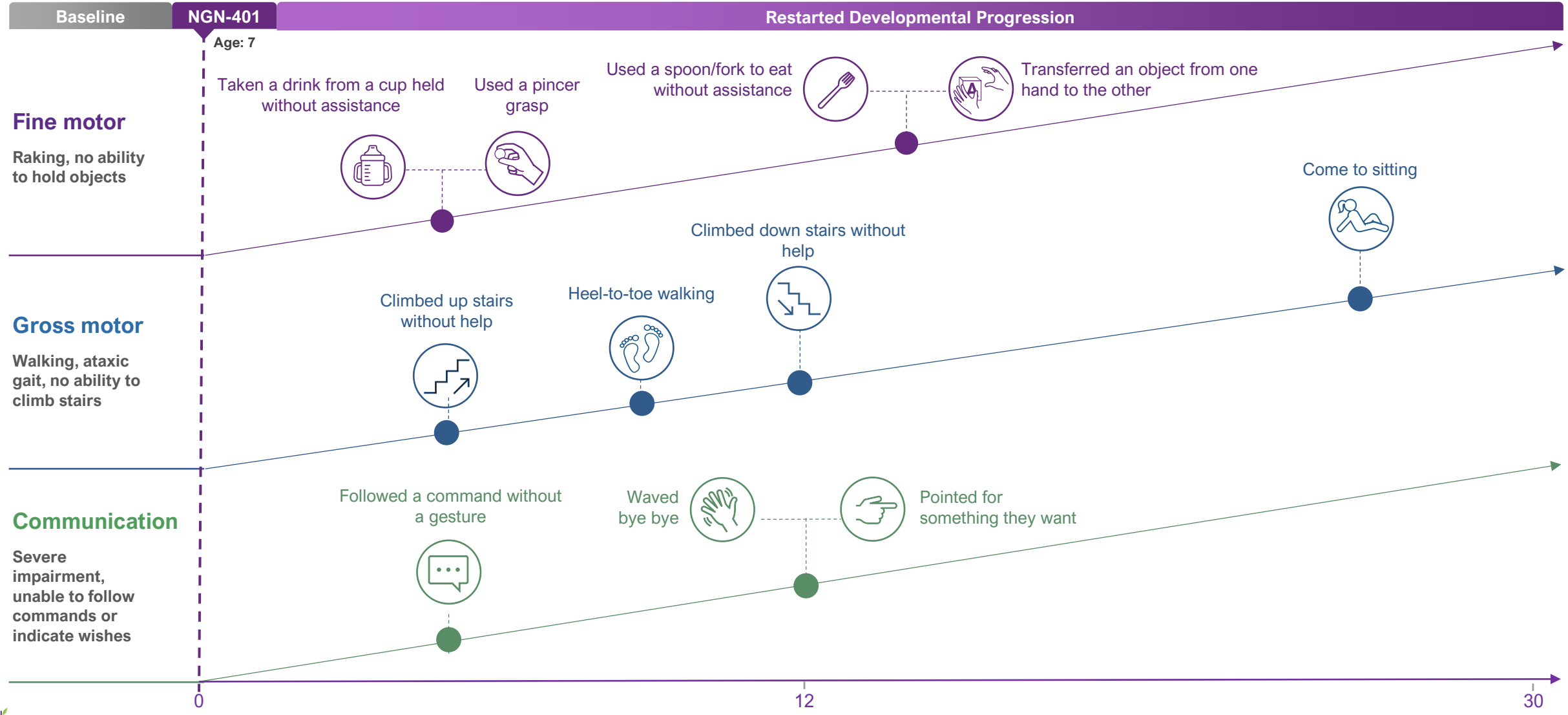


Restart of developmental trajectory across multiple domains can lead to increased independence and reduced caregiver burden

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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



As of data cutoff date of June 16, 2026

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

At baseline (Age 7):

- **Impaired hand use**, raking, no ability to hold objects
- **Gross motor impairment,** ataxic, unable to climb stairs
- **Severe communication impairment,** unable to follow commands

Moves more independently at home



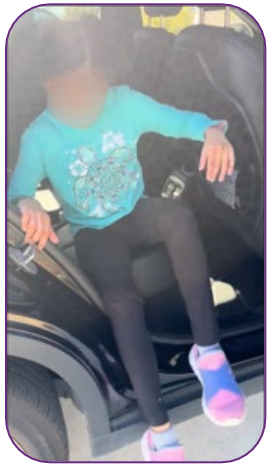
Gets in/out of bathtub, on/off furniture & up/down stairs, without help

Joins family outings



Shops with Mom, carrying the basket with both hands

Navigates the world independently



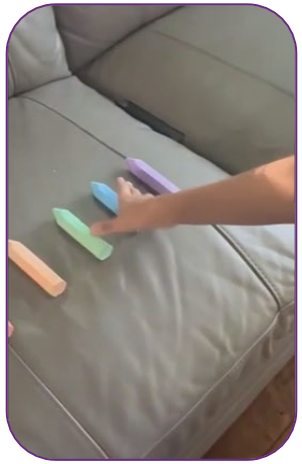
Climbs in & out of the car & shuts the door herself

Follows multi-step directions



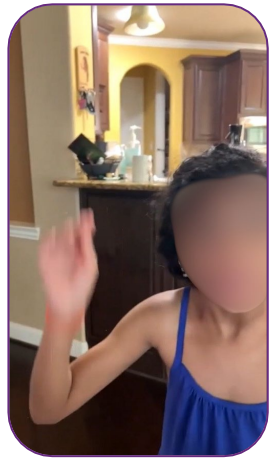
Carries her backpack, takes the stairs, & closes the door – following directions in 2 languages

Follows instructions to pick the right colors



Uses hands to select correct colors when asked

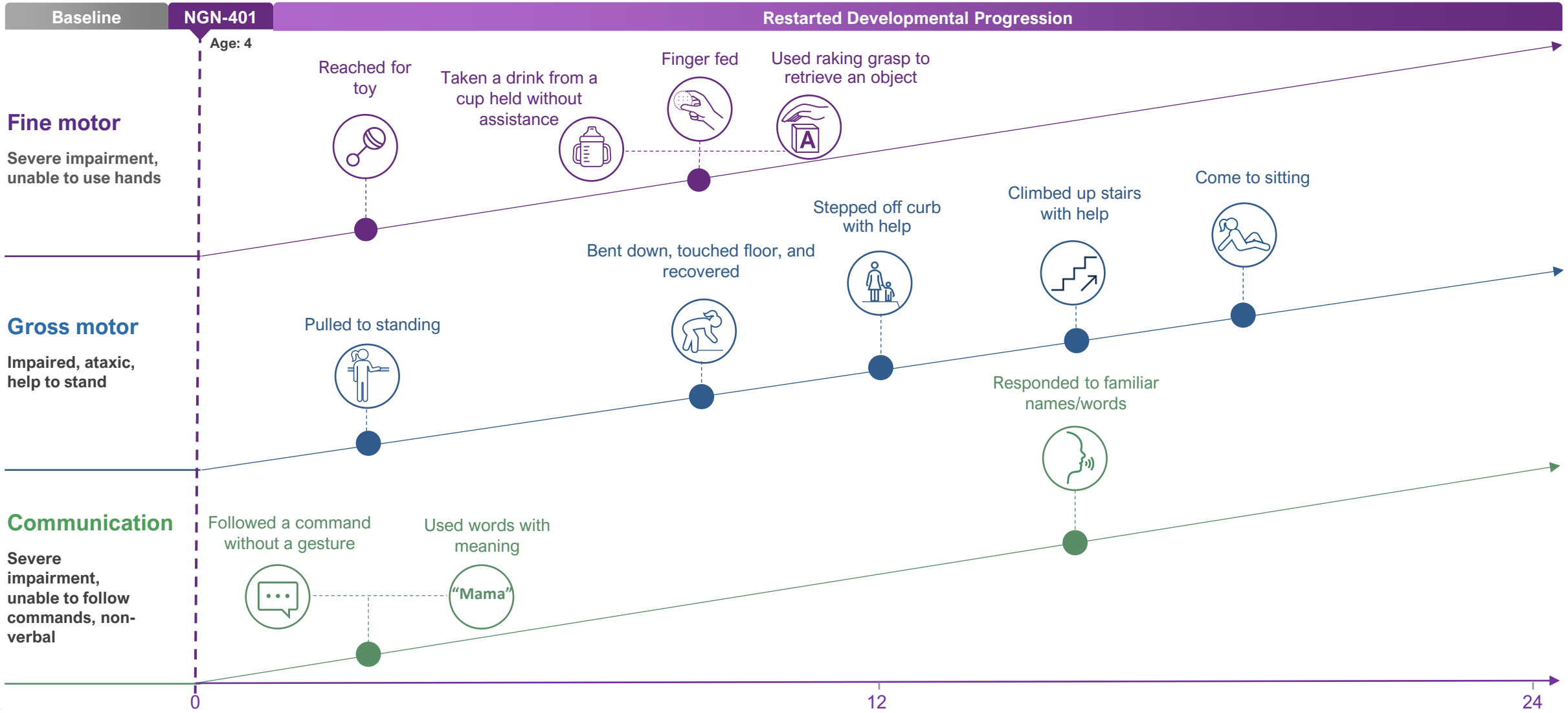
Greets family in context



Waves hello to greet people on video calls

Enhanced Independence and Reduced Caregiver Burden Post-NGN-401

Pt:2 Developmental Milestones Gained in Ordered, Developmental Progression



As of data cutoff date of June 16, 2026

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

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

Less caregiver assistance at mealtime



Self-feeds and holds own drinks

Moves more independently with less supervision



Bends down to pick up toys & blankets without help

Connects with family



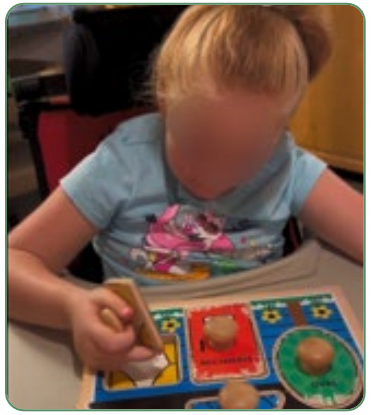
Turns when called, says words with meaning & follows instructions

Enhanced Independence and Reduced Caregiver Burden Post-NGN-401

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

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

Uses both hands to follow instructions



Follows directions in two languages and chooses the correct puzzle piece

Stands and moves with less help



Requires less physical support to stand and move

Feeds herself



Self-feeds using a pincer grasp

Enhanced Independence and Reduced Caregiver Burden Post-NGN-401

As of data cutoff date of June 16, 2026

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

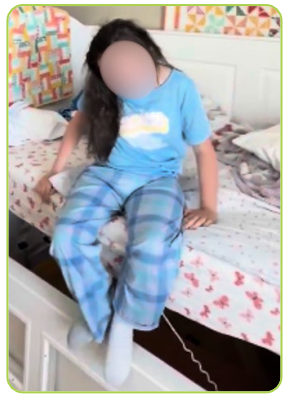
At baseline (Age 7): **Impaired hand use,** raking grasp; **Severe gross motor impairment,** cannot sit/stand/walk independently; **Severe communication impairment,** unable to follow commands

Eats on her own



Feeds herself with a regular spoon — easier, more independent mealtimes

Sits up by herself



Pushes herself up from lying down, moving through her day with more freedom

Controls her space



Turns the lights on and off herself, taking charge of her surroundings

Plays with Mom with both hands



Catches a ball with both hands and plays back-and-forth with others

Joins in family play



Plays with balloons at a family celebration, demonstrating her new hand use

Makes her own needs known



Tells her caregiver what she wants more reliably using her communication device

Connects with family

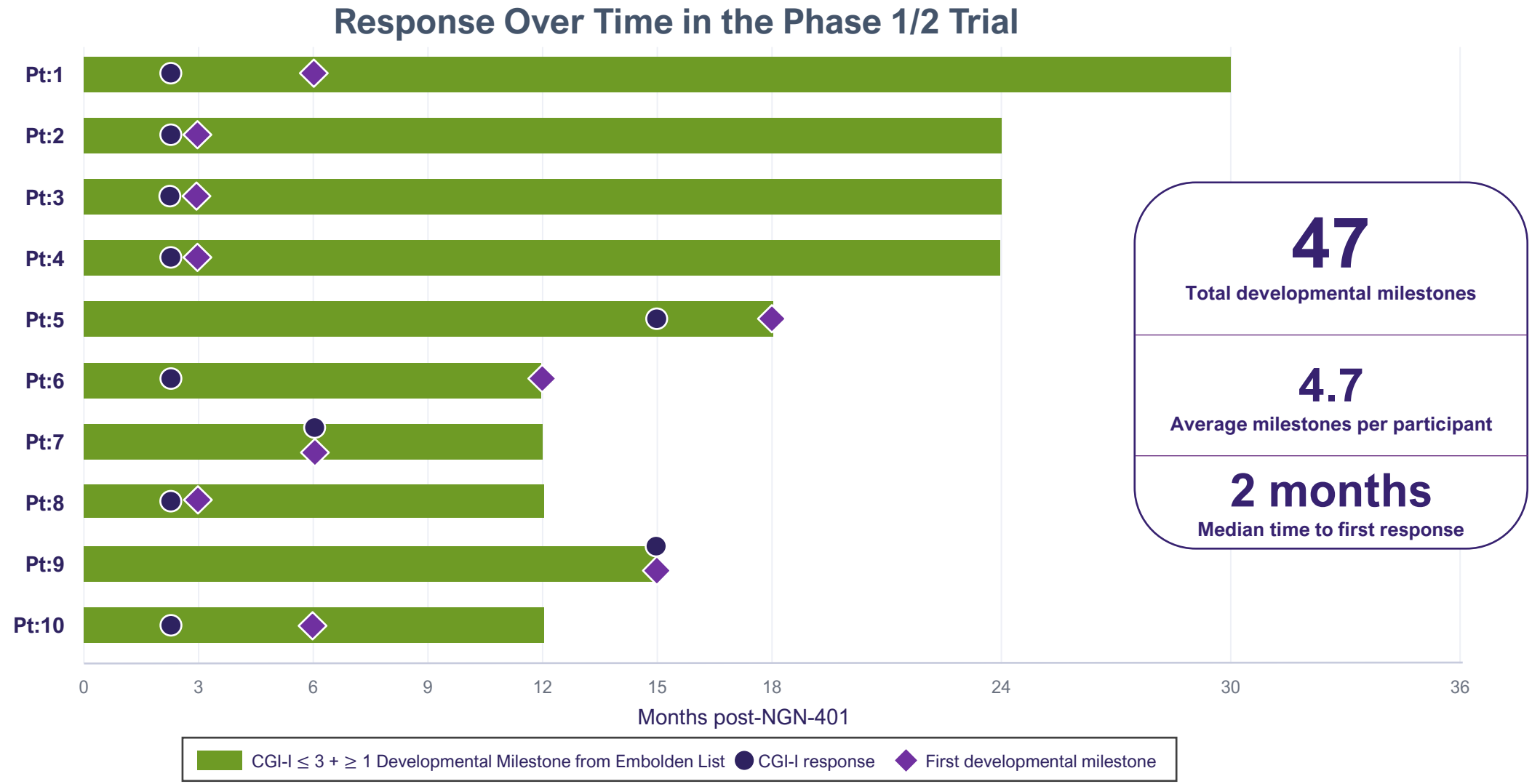


Shares more moments with family, including giving “high fives”

Enhanced Independence and Reduced Caregiver Burden Post-NGN-401

As of data cutoff date of June 16, 2026

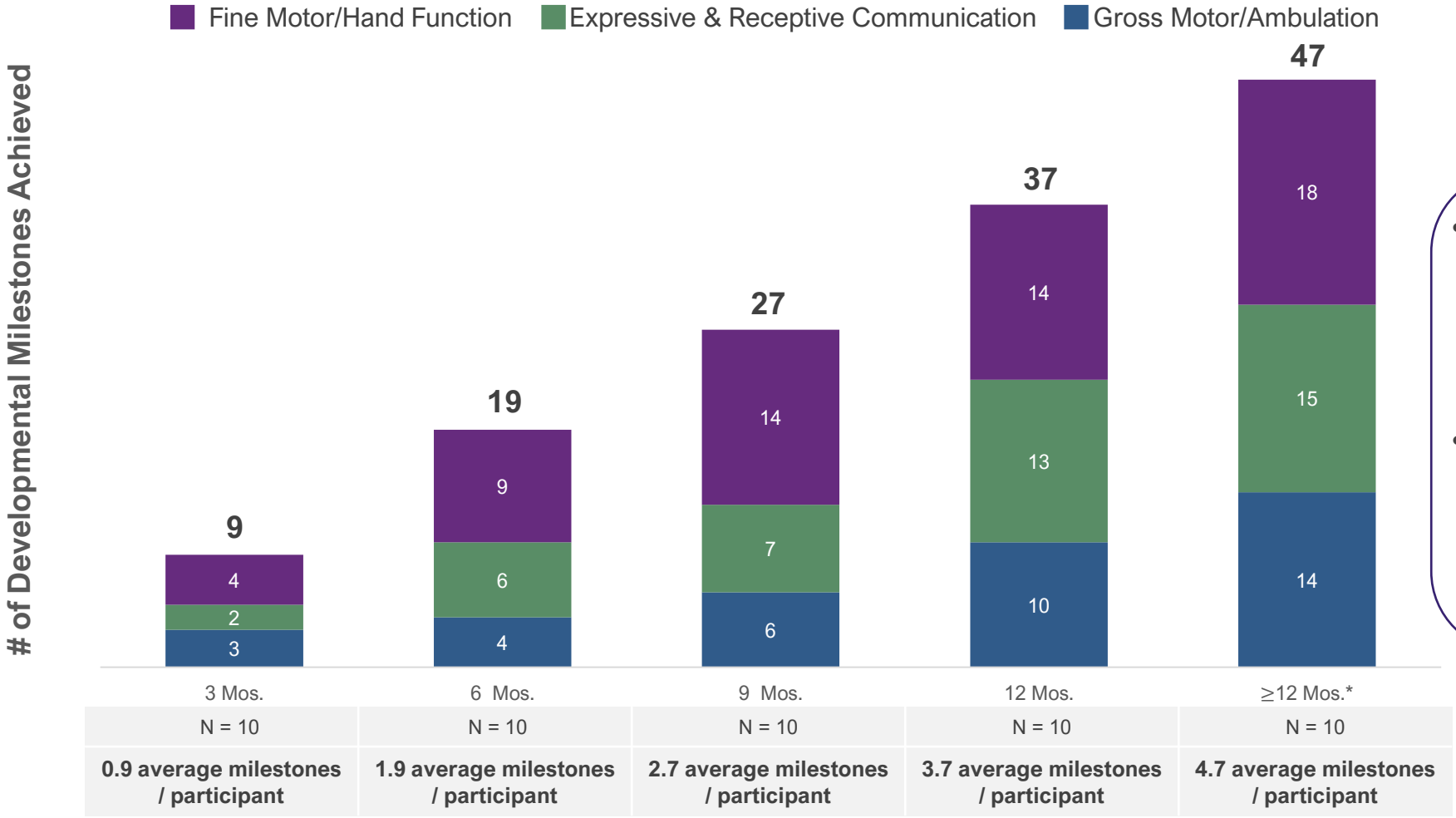
Rapid Response Observed Post NGN-401 that Deepened Over Time



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



- **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

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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

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

Embolden
A Clinical Study for Rett Syndrome

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

Screening Period
(Up to 45 days)

Primary Observation (12 Months)

NGN-401
One-time 1E15 vg dose
Patients ≥ 3 years

Total participants dosed: N=25

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**

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

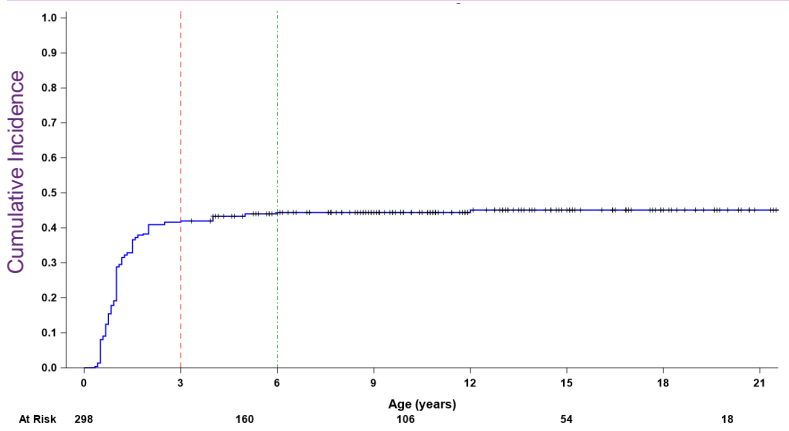
Natural History Study Cumulative Incidence Model Shows Beyond Age 3, Milestone Gains Are Rare with Minimal Difference at Age 6

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

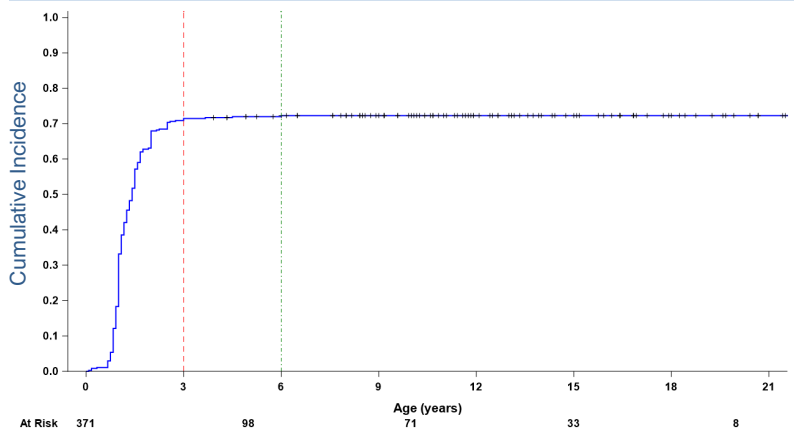
% ever gained/regained: **46%** | At ≥3 years: **4.2%** | At ≥6 years: **1.3%**



Gross Motor

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

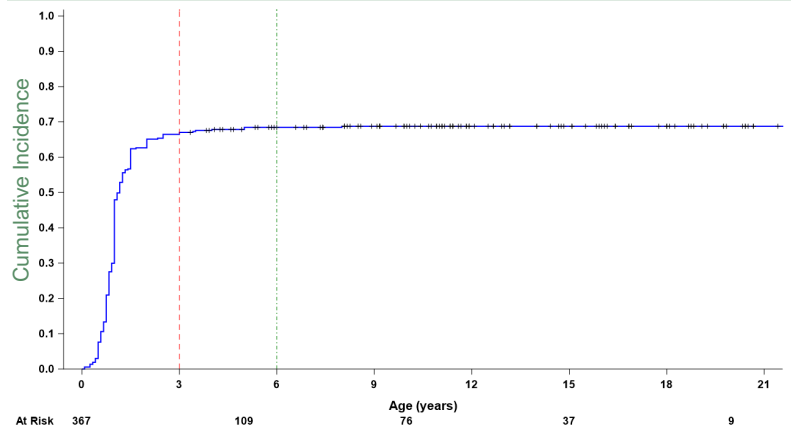
% ever gained/regained: **72%** | At ≥3 years: **1.5%** | At ≥6 years: **0.3%**



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%**

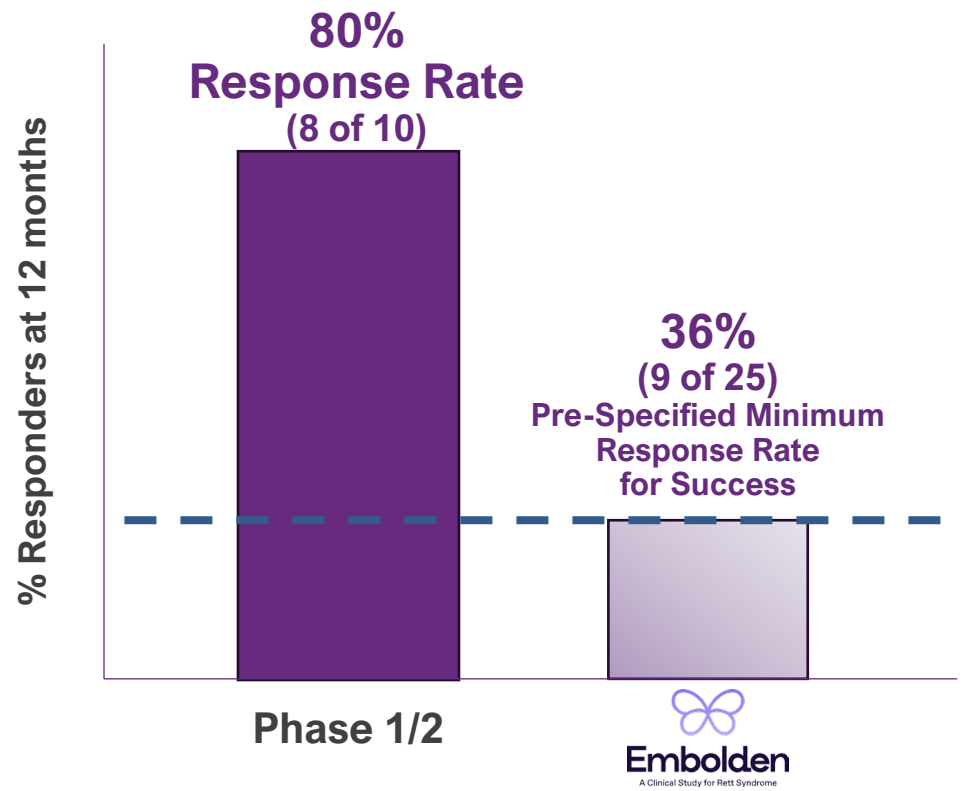
Ex: Participant **Age 14**
2 milestones gained
Age-matched RNHS analysis cumulative incidence rate: **0.0%**

As of data cutoff date of June 16, 2026
IRSF Scientific Meeting 2026
RNHS: Rett Syndrome Natural History Study

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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

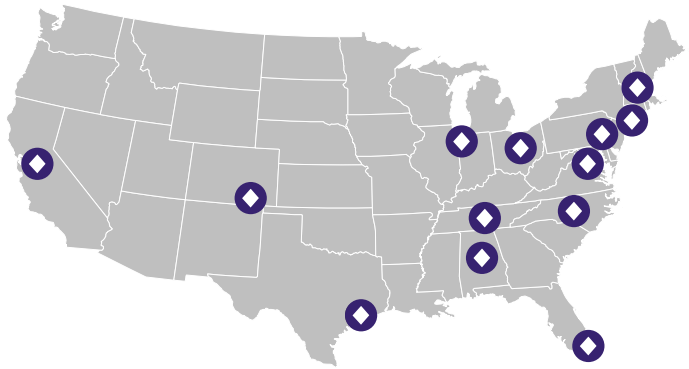
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As of data cutoff date of June 16, 2026

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

APPENDIX: ADDITIONAL NGN-401 DATA

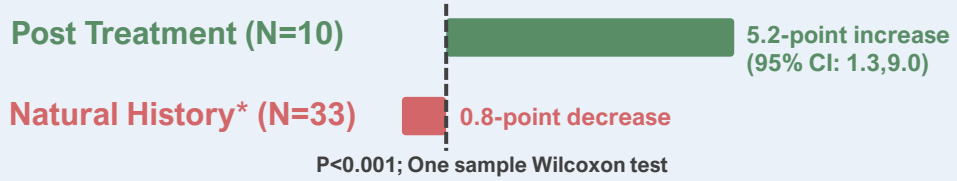
RSGMS Phase 1/2 Data Showed Meaningful Gains in Gross Motor Function

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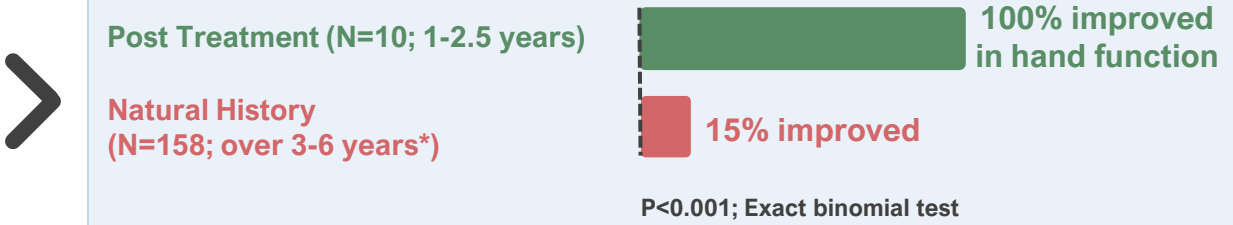
RSHFS Phase 1/2 Data Showed Meaningful Gains in Hand Function

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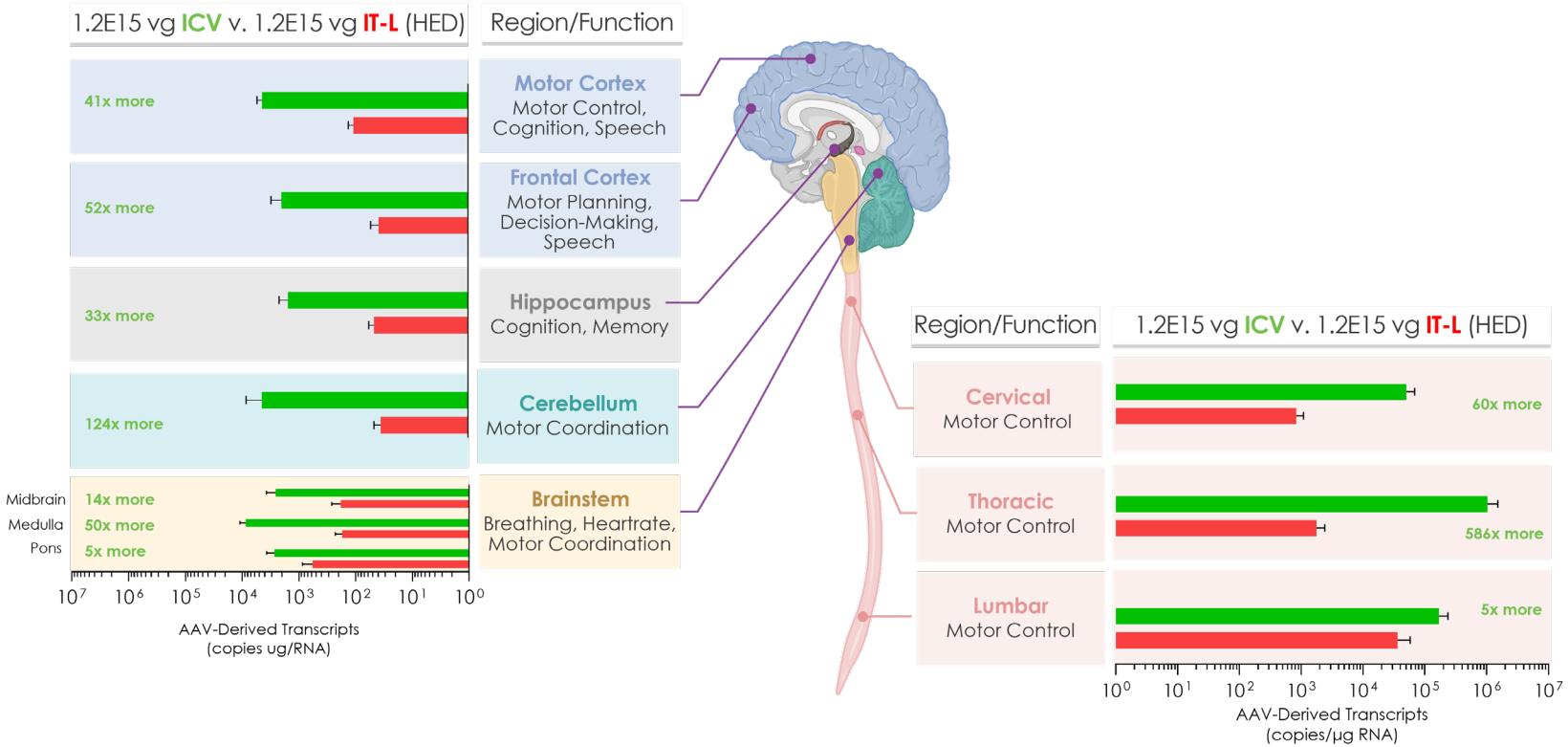
*Downs J *et al. J Pediatr* 2021; 237: 244–249; PMID: 34214590

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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

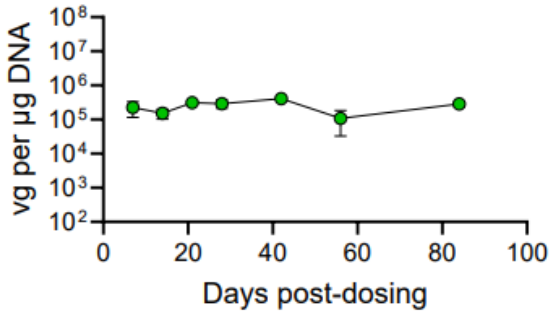
An estimated 30,000 ICV procedures are performed by neurosurgeons annually in the U.S. and require minimal downtime/recovery



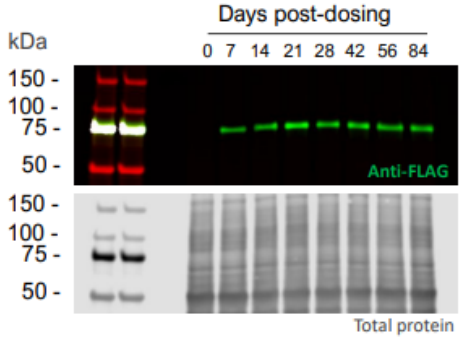
ICV = Intracerebroventricular
IT-L = intrathecal lumbar
ESGCT 2025

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

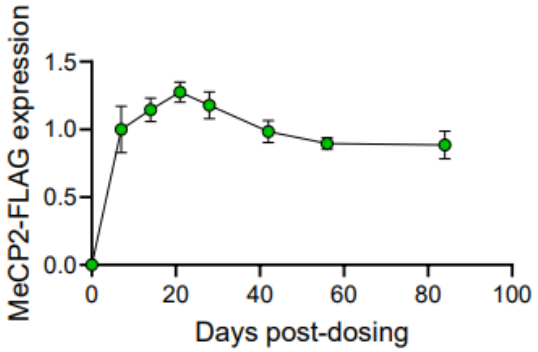
ssAAV is required to accommodate full-length *MECP2* gene; ssAAV-mediated MeCP2 expression detectable by day 7 post dosing and sustained for the 3-month duration of the study



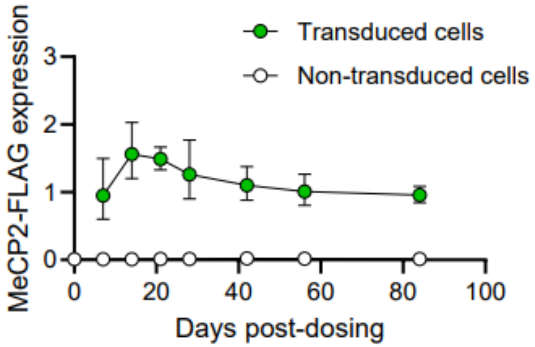
Quantification of vg in neocortex



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