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Restarting Development in Rett Syndrome

NGN-401 for Rett Syndrome

Phase 1/2 Update

June 29, 2026

Disclaimer

Forward Looking Statements

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Today's Speakers

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

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

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

Introduction

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

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

The Unmet Need

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

The Market

15,000–20,000
patients — US, EU & UK

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

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

52% Pediatric	48% Adult

All living with profound unmet need

The Opportunity

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

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

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

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

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

100%

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

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

47

total milestones gained

4.7 average milestones per participant

30

months of **continued improvement post-dose**

no plateau observed – no milestones lost

Restarted Development

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

Real-World Impact

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

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

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

Topline Results
Expected 2H 2027

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

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

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

As of data cutoff date of June 16, 2026

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Phase 1/2 Data Update

NGN-401 Clinical Study Design for Treatment of Rett Syndrome

Phase 1/2 converted into a single registrational study

Study Design

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

Population Eligibility Criteria

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

Key Clinical Assessments

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

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)
Primary efficacy population (N=24)
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

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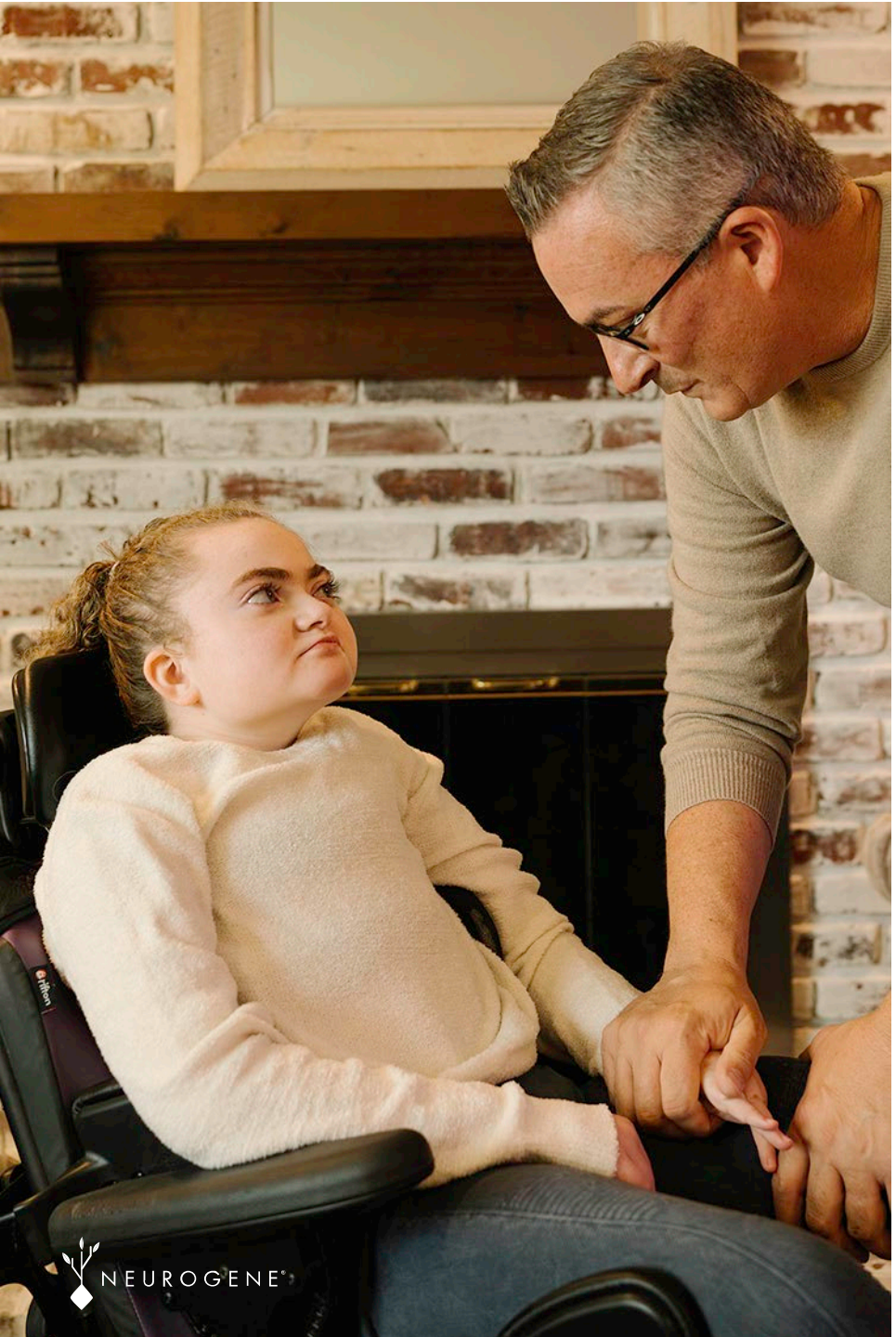
As of data cutoff date of June 16, 2026
Pt = Participant

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

Bernhard Suter, M.D.

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

Principal Investigator in NGN-401 Clinical Trial



Rett Syndrome: Rare, Debilitating, Progressive, Neurodevelopmental Disorder

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

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

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

Hallmark features:

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

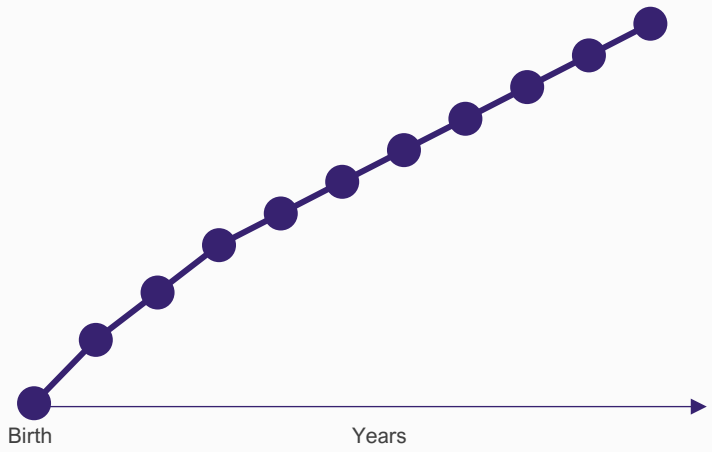
Living with Rett Syndrome

Kendall & Kati

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

Typical Development

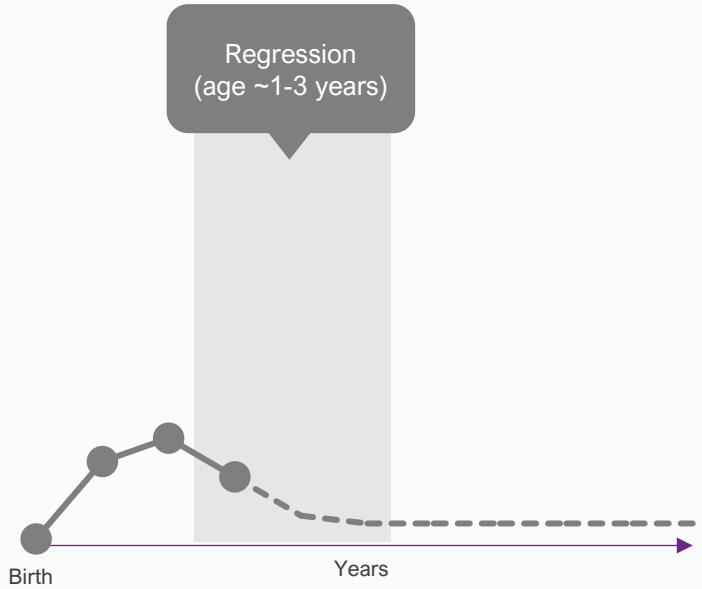
Organized, cumulative acquisition of developmental milestones



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

Rett Syndrome

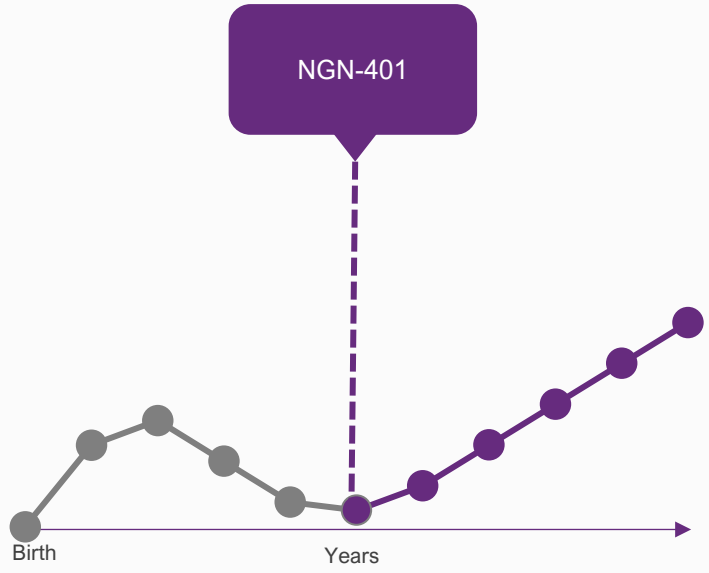
Milestones lost in regression, exceedingly rare to gain milestones post-regression



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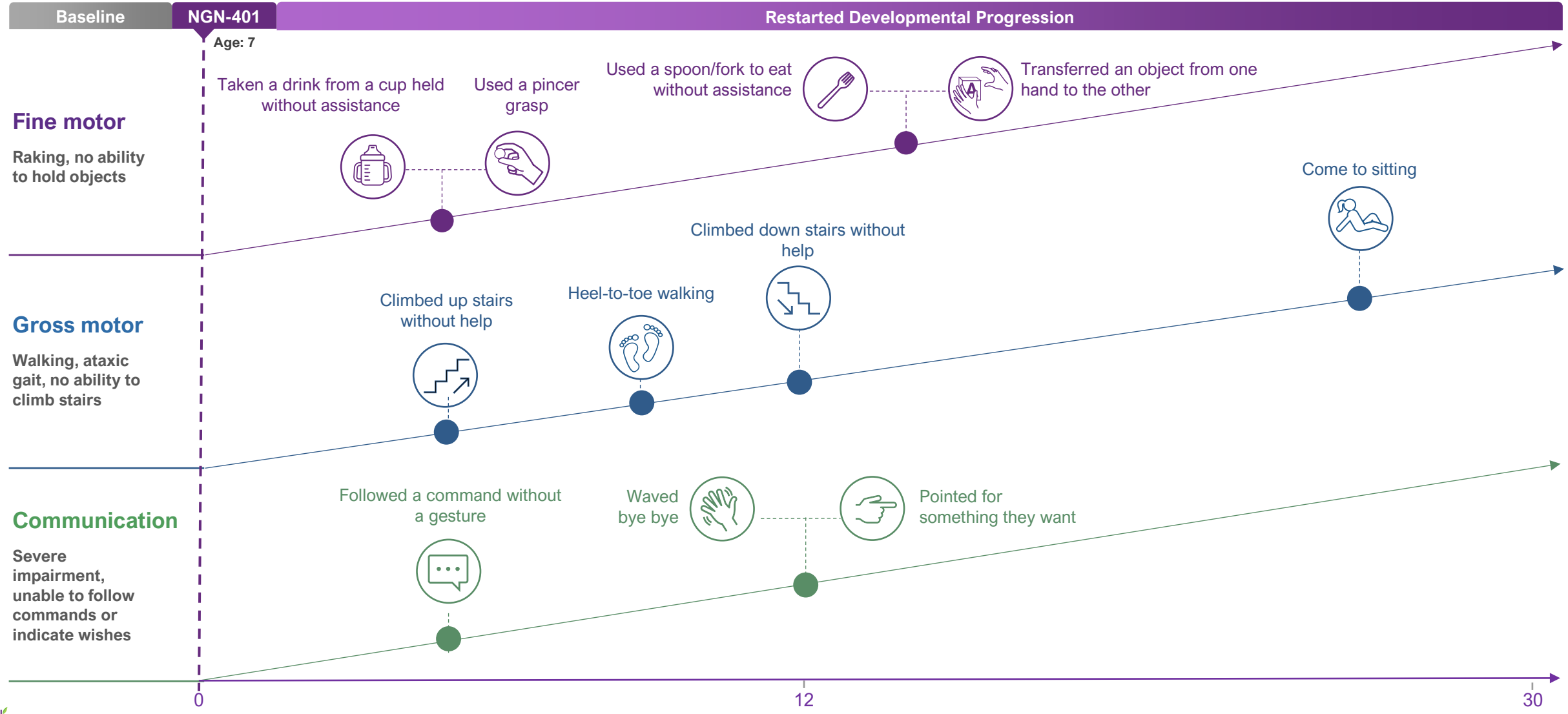
Frankenburg et al., Denver II Technical Manual May D, *et al*. *J Neurodev Disord*. 2024;16(1):42
Graphs for illustrative purposes only

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

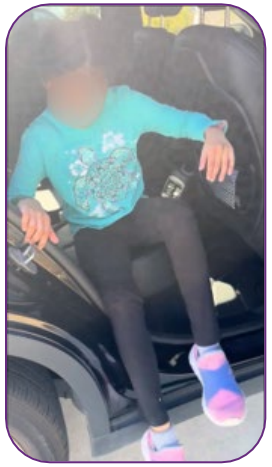
Moves more independently at home

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

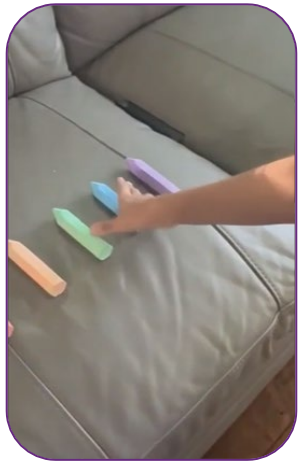


Climbs in & out of the car & shuts the door herself

Follows multi-step directions

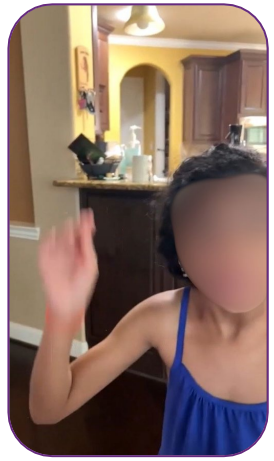
Carries her backpack, takes the stairs, & closes the door – following directions in 2 languages

Follows instructions to pick the right colors



Uses hands to select correct colors when asked

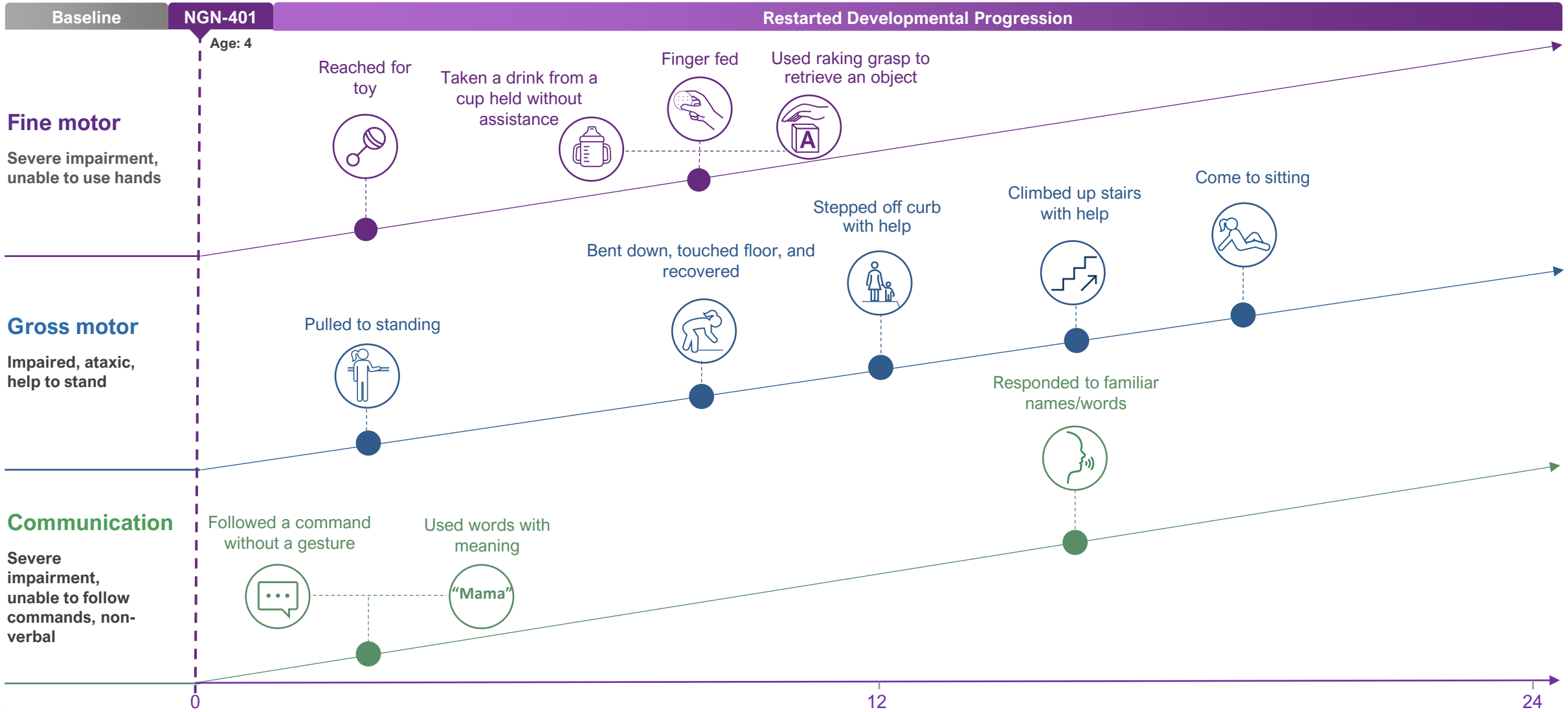
Greets family in context



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

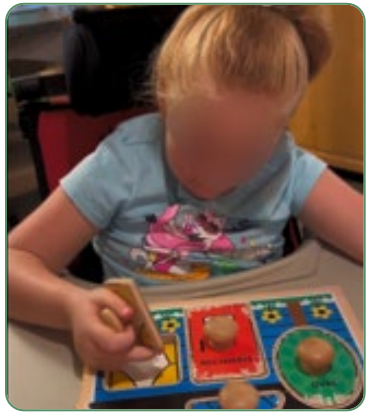
Turns when called, says words with meaning & follows instructions

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

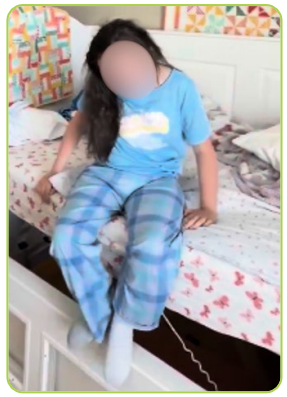
Feeds herself

Self-feeds using a pincer grasp

Enhanced Independence and Reduced Caregiver Burden Post-NGN-401

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

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

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

Enhanced Independence and Reduced Caregiver Burden Post-NGN-401

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Thank you to all participants and their families and caregivers!

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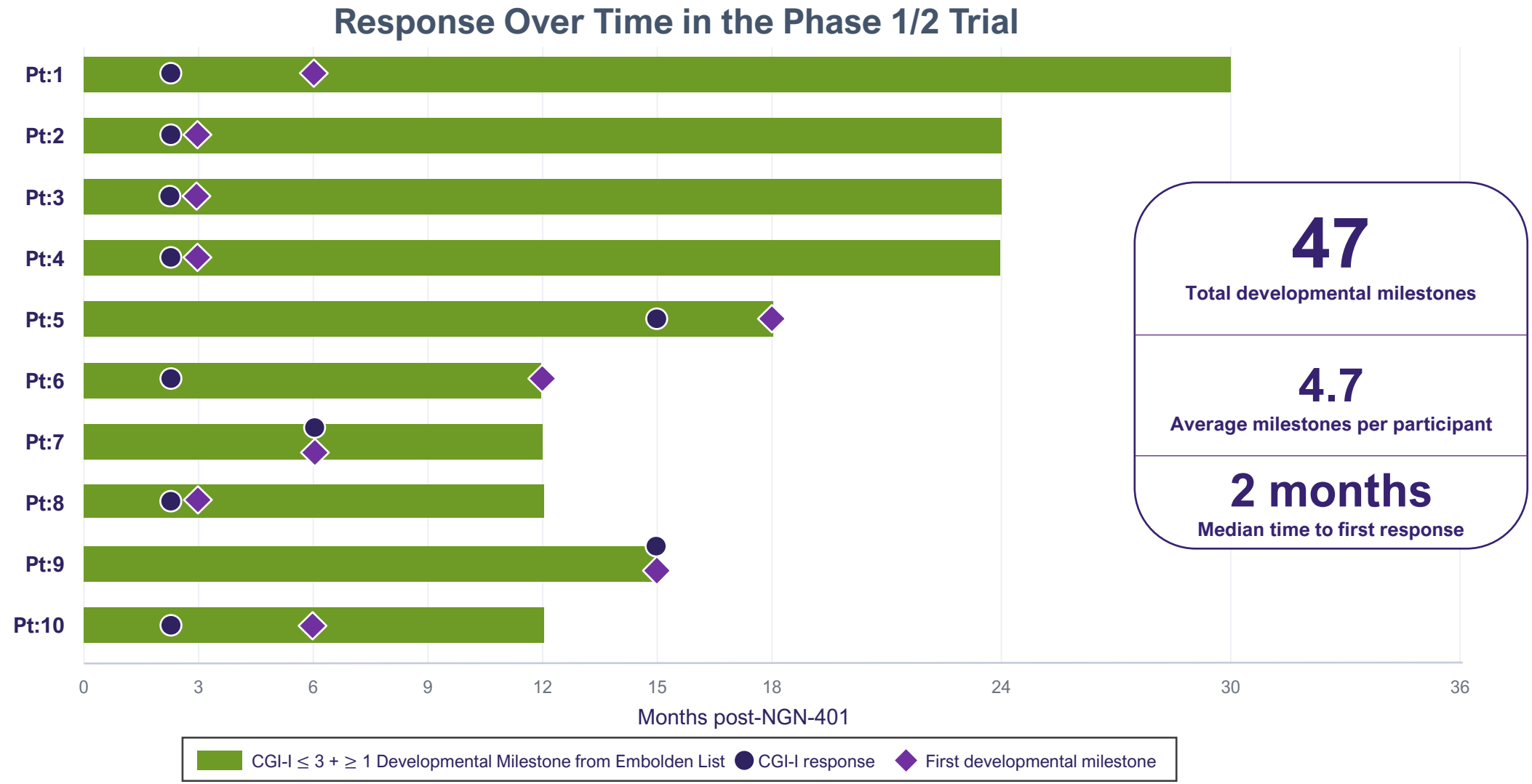
Phase 1/2 Data Update

Julie Jordan, M.D.
Chief Medical Officer

Rapid Response Observed Post NGN-401 that Deepened Over Time



47
Total developmental milestones

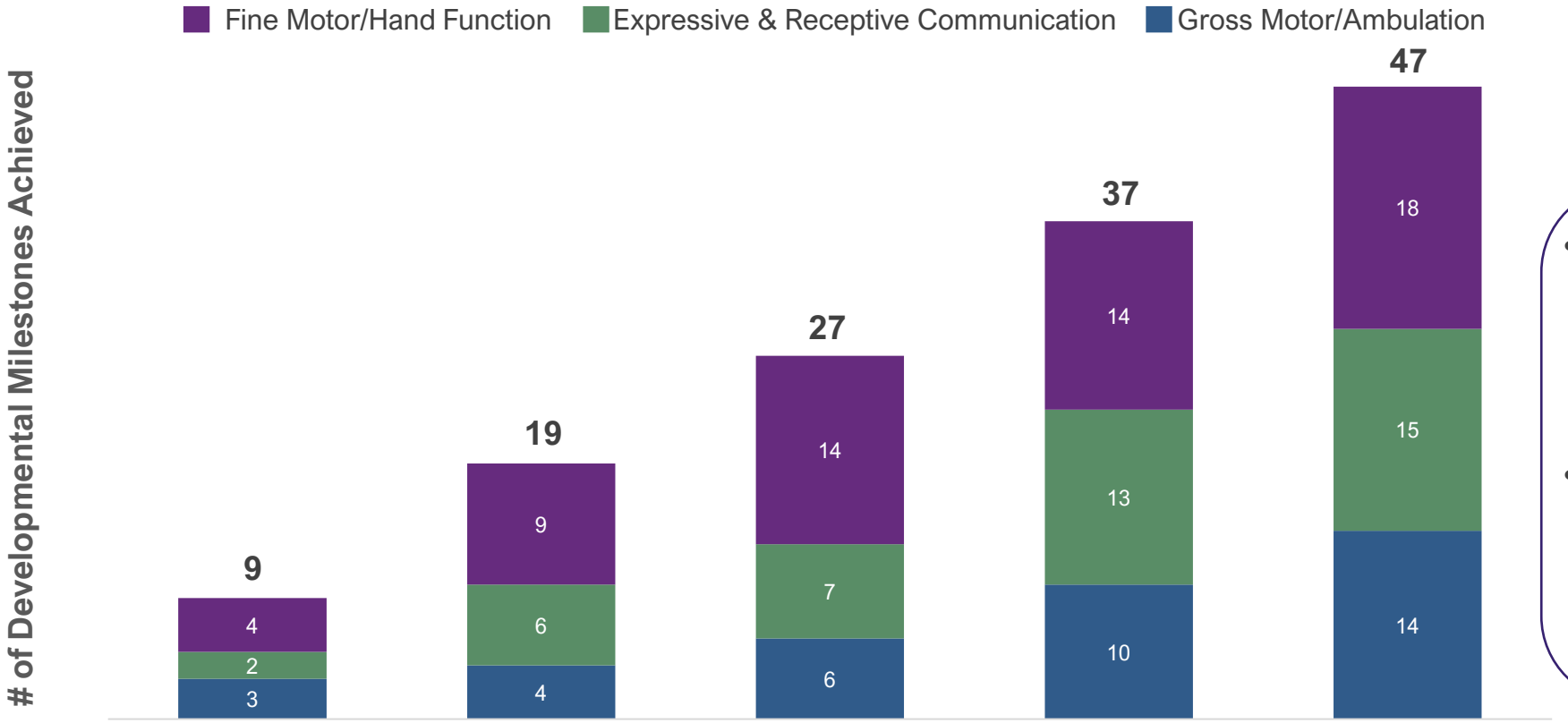
4.7
Average milestones per participant

2 months
Median time to first response

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

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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

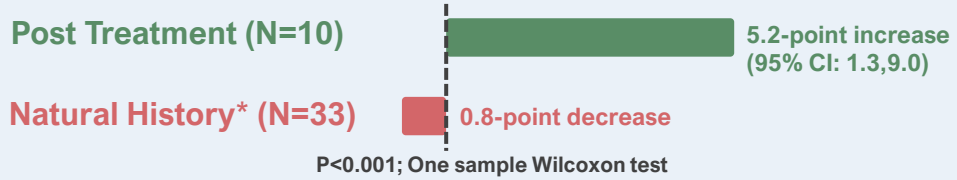
RSGMS Phase 1/2 Data Showed Meaningful Gains in Gross Motor Function

Overview of Rett Syndrome Gross Motor Scale (RSGMS)

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

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

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



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

7 unable to perform independently at baseline

SIT TO STAND

Reduces physical burden on caregiver, enhances independence

6 ambulatory unable to perform independently at baseline

STAND UP FROM FLOOR

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

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As of data cutoff date of June 16, 2026

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

IRSF Scientific Meeting 2026

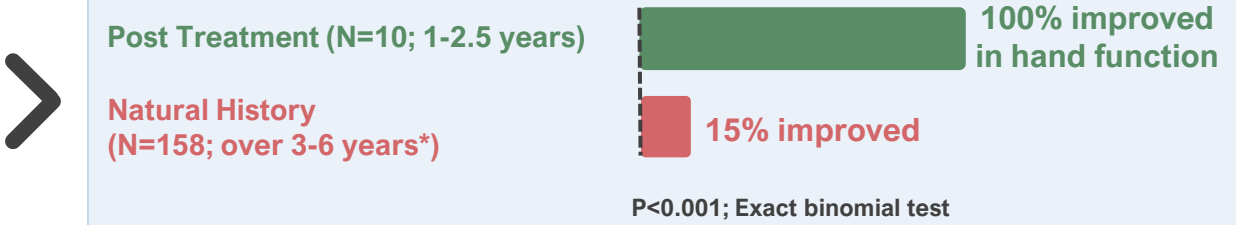
RSHFS Phase 1/2 Data Showed Meaningful Gains in Hand Function

Overview of Rett Syndrome Hand Function Scale (RSHFS)

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

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

Mean RSHFS Total-Score Gains Exceed Natural History



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

10/10 improved

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

GRASPING, PICKING UP, HOLDING LARGE OR SMALL OBJECTS

Enhances ability to self-feed, increases independence

1.8 point gain per hand

7/9 bilateral improvement

N=9 at baseline had significant hand function impairment

MORE COMPLEX ACTIVITIES

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

As of data cutoff date of June 16, 2026

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

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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TEAE = Treatment emergent adverse event

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Embolden
A Clinical Study for Rett Syndrome

Embolden Registrational Trial

Dosing Completed in Embolden Registrational Trial to Support Planned BLA Submission

Embolden
A Clinical Study for Rett Syndrome

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

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 efficacy population: N=24

Primary Endpoint at 12 Months

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

33% response rate, or 8 of 24 participants, needed for success

Key Secondary Endpoints

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

Developmental Milestones

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

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

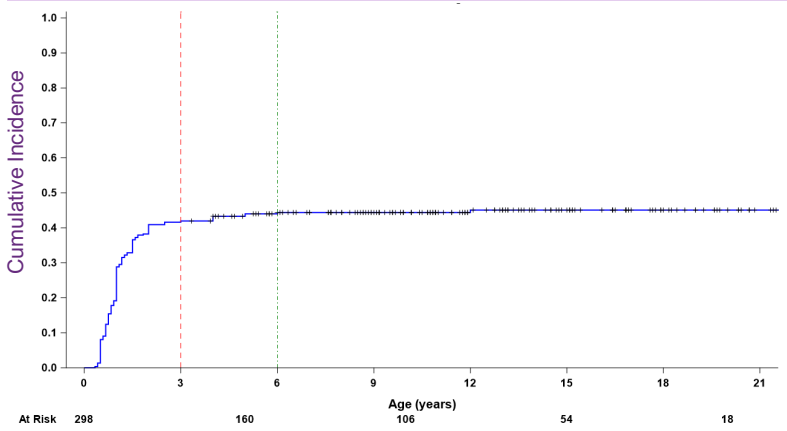
Natural History Study Cumulative Incidence Model Shows Beyond Age 3, Milestone Gains Are Rare with Minimal Difference at Age 6

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

Fine Motor

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

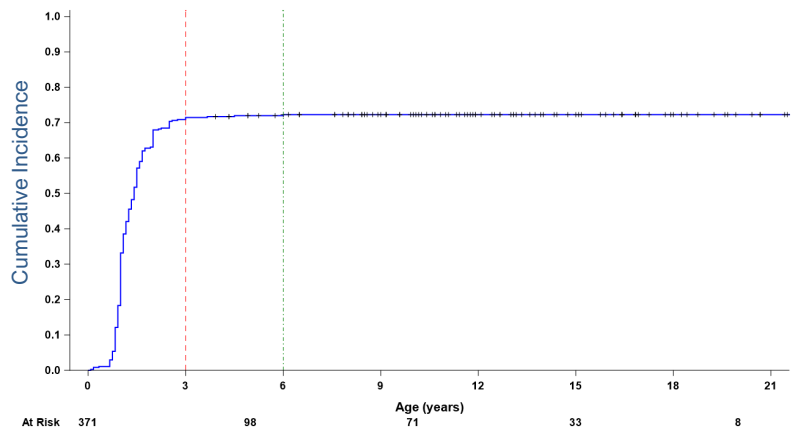
% ever gained/regained: **46%** | At ≥3 years: **4.2%** | At ≥6 years: **1.3%**



Gross Motor

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

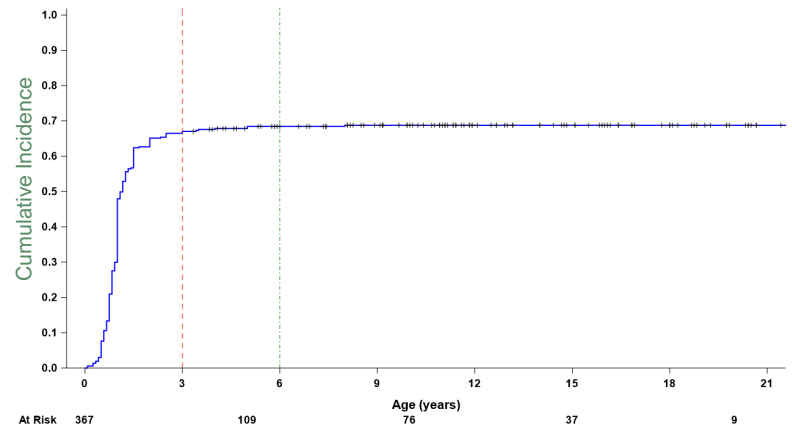
% ever gained/regained: **72%** | At ≥3 years: **1.5%** | At ≥6 years: **0.3%**



Communication

Example Milestone: Used words with meaning

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



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

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

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

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

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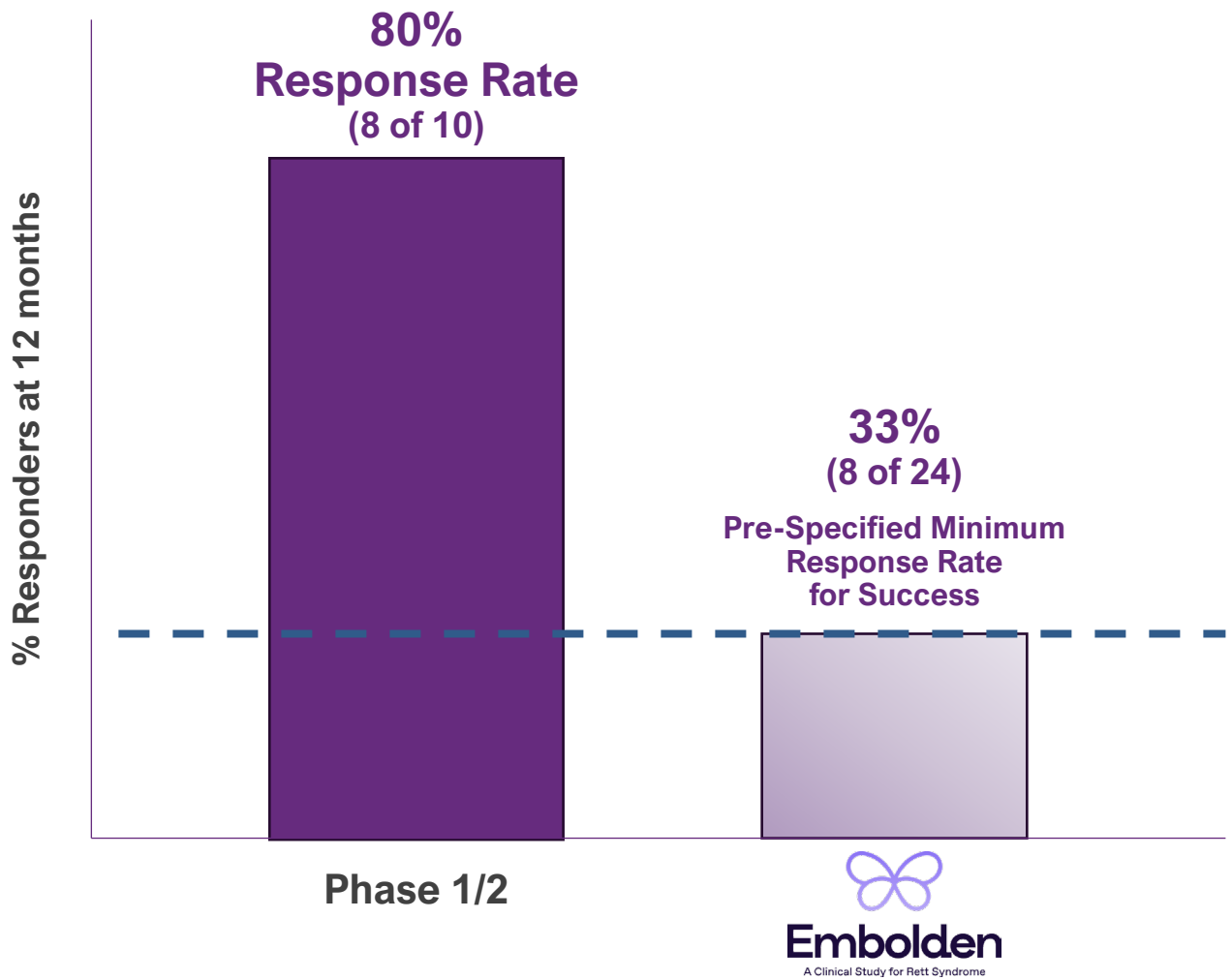
IRSF Scientific Meeting 2026

Compelling Clinical Data Strengthen Confidence in Potential Embolden Registrational Success



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

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Advancing NGN-401 Towards Commercialization

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

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

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

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

Embolden fully dosed, topline data expected 2H 2027

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NGN-401 is Poised to Transform Treatment for Rett Syndrome

TODAY

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

UPCOMING ANTICIPATED MILESTONES

- **Start PPQ Campaign Mid-2026**
- **Topline Data from Embolden in 2H 2027**
- **BLA Submission for NGN-401**
- **Continue Additional Commercial-Readiness Activities**

Strong Cash Balance (1Q'28)

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

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Thank you to all participants, caregivers, families, investigators, clinical site coordinators, study coordinators, site staff and the Rett syndrome community for your trust, partnership and ongoing support of the development of NGN-401!