Bringing New Treatments to Life

Corporate Presentation | May 2026

Taysha GENE THERAPIES

Legal disclosure

FORWARD LOOKING STATEMENTS

This presentation contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this presentation, including statements regarding the potential of TSHA-102, the durability and reproducibility of the clinical data from the REVEAL trials, the anticipated Part B trial design, our research, development and regulatory plans, and our strategy, future operations, communications with and feedback from the FDA and Health Canada on the regulatory pathway for TSHA-102, future financial position and cash runway, future revenues, projected costs, prospects, plans, objectives of management, and our ability to successfully commercialize our product candidates are forward-looking statements. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements are subject to a number of risks, uncertainties and assumptions. Risks regarding our business are described in detail in our Securities and Exchange Commission filings, including in our Annual Report on Form 10-K for the year ended December 31, 2025, and our other filings with the SEC, which are available on the SEC’s website at www.sec.gov. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. The forward-looking statements contained in this presentation reflect our current views with respect to future events, and we assume no obligation to update any forward-looking statements except as required by applicable law.

This presentation includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties as well as our own estimates of potential market opportunities. All of the market data used in this presentation involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such data. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. Our estimates of the potential market opportunities for our product candidates include several key assumptions based on our industry knowledge, industry publications, third-party research and other surveys, which may be based on a small sample size and may fail to accurately reflect market opportunities. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions.

Key investment highlights

TSHA-102: potential one-time treatment designed to address root cause of Rett syndrome, with a clear path to registration

High Unmet Need and Significant Market Opportunity	○ No approved therapies address genetic root cause of Rett syndrome ○ 15,000-20,000 patients (U.S., EU+U.K.); 1 of 10,000 female births worldwide[1]
Differentiated Approach Designed to Support Commercial Uptake	○ Clinically and commercially proven AAV9 capsid with well-established safety profile, based on third-party gene therapy studies ○ Delivered intrathecally as a routine, minimally invasive procedure with outpatient potential, enabling broad, scalable access
Pivotal Trial Design Enabled by Natural History Data	○ Patients aged ≥6 years are in developmental plateau, with a 0% to <6.7% likelihood of achieving a developmental milestone[2] ○ Single-arm, open label trial, enrolling 15 patients with patient serving as own control, assessing the % of patients aged 6 to <22 years (developmental plateau population) who gain/regain ≥one developmental milestone as the primary endpoint
Advancing with Clear Path Toward Registration	○ Finalized FDA alignment on REVEAL pivotal trial protocol and SAP; 6-month interim may serve as basis for BLA submission ○ Written FDA alignment on inclusion of ≥3 months of ASPIRE safety data (N=3, aged 2 to <4 years) in BLA submission to support a broad ≥2 years label
Transformative Potential Supported by Part A Data[3]	○ 100% response rate in REVEAL Part A for pivotal trial primary endpoint exceeds minimum threshold for success of 33%[4] ○ No treatment-related SAEs or DLTs following low and high dose of TSHA-102 ○ FDA Breakthrough Therapy designation granted to TSHA-102

[1]Amir, R E et al. "Rett syndrome is caused by mutations in X-linked MECP2, encoding methyl-CpG-binding protein 2." *Nature genetics* vol. 23,2 (1999): 185-8. doi:partners. 10.1038/13810. (estimated prevalence of *15,000-20,000 patients with typical Rett syndrome caused by a MECP2 mutation*). [2]Neul, Jeffrey L et al. "Trajectory of skill acquisition, loss, and regain in females with classic Rett syndrome." Journal of neurodevelopmental disorders, 10.1186/s11689-026-09680-6. 12 Mar. 2026, doi:10.1186/s11689-026-09680-6. [3]Efficacy data based on May 19, 2025, data cutoff (N=10); Safety data based on May 2026 data cutoff (N=12). Study is ongoing and data is subject to change. [4]Minimum threshold per REVEAL pivotal trial SAP. CNS=Central nervous system; SAP=Statistical analysis plan; BLA=Biologics license application; SAE=Serious adverse event; DLT=Dose-limiting toxicity

TSHA-102 Rett syndrome program strongly positioned for rapid execution toward BLA submission

Compelling Part A Clinical Data

supported FDA Breakthrough Therapy designation and indicates pivotal trial is well powered to establish efficacy

Initiated BLA-enabling PPQ Campaign

FDA alignment on PPQ and comparability strategy may enable pooling Phase 1/2 and pivotal data for BLA submission

Potential for Expedited Path to BLA Submission

FDA alignment that REVEAL pivotal trial 6-month interim analysis may serve as the basis for BLA submission

Potential for Broad Label Aged 2+ Years

FDA alignment on inclusion of ≥3 months of ASPIRE safety data in BLA submission to support a broad label

Next Steps

Completion of dosing in REVEAL pivotal trial and ASPIRE trial expected **Q2 2026**

Longer-term clinical data from Part A REVEAL Phase 1/2 trials expected **Q2 2026**

Completion of BLA-enabling PPQ campaign expected **Q4 2026**

BLA=Biologics license application; PPQ=Process Performance Qualification

Commercial readiness activities designed to position TSHA-102 for successful launch and adoption

Seasoned Commercial Leadership Team

Deep expertise across gene therapy commercialization, market development and market access

Blockbuster gene therapy launch experience (Zolgensma, AveXis)

Market Research Supports Potentially Strong Demand and Broad Adoption[1]

High clinician intent to treat across all ages, with 90% of caregivers likely to pursue gene therapy upon approval

Strong preference for IT delivery over direct-to-brain due to familiarity, accessibility and scalability

Intrathecal Administration Enables Broad Access

Routine outpatient procedure enables scalable access beyond COEs to regional and local institutions

~75% of patients are managed outside large Rett COEs[2]

[1]2026 market research conducted by a third party with U.S. clinicians and caregivers of individuals with Rett syndrome. [2]Based on U.S. claims data
IT=intrathecal; COEs=centers of excellence

Rett syndrome: a devastating, rare and progressive X-linked neurodevelopmental disease

- Primarily occurs in females
- Caused by mutations in the X-linked gene encoding MeCP2 protein, which inhibits neuronal developments[1]
- Leads to impaired brain development and function, resulting in multisystem complications[1]
- Symptoms and severity vary due in part to random X-inactivation[2]

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[1]Amir, R E et al. "Rett syndrome is caused by mutations in X-linked MECP2, encoding methyl-CpG-binding protein 2." *Nature genetics* vol. 23,2 (1999): 185-8. doi:10.1038/13810 [2]Braunschweig, Daniel et al. "X-Chromosome inactivation ratios affect wild-type MeCP2 expression within mosaic Rett syndrome and Mecp2-/+ mouse brain." *Human molecular genetics* vol. 13,12 (2004): 1275-86. doi:10.1093/hmg/ddh142.

Hallmark characteristics of Rett syndrome appear across multiple clinical domains **impacting activities of daily living**

Results in **loss of functional abilities and developmental milestones** and the emergence of hallmark symptoms by 6 years that progress over time[1]

Gross Motor Function

- Mobility issues
- Loss of movement and coordination abilities
- Gait disturbances
- Hypotonia
- Dystonia

Fine Motor Function

- Loss of hand function
- Loss of purposeful hand use
- Repetitive hand movements

Communication / Socialization

- Loss of speech / communication
- Social withdrawal
- Behavioral issues
- Intellectual disability

Autonomic Function / Seizures

- Epilepsy
- Sleep disturbances
- Breathing issues
- Gastrointestinal issues
- Cardiac dysfunction
- Vasomotor disturbances

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[1]Gold, Wendy A et al. "Rett Syndrome: A Genetic Update and Clinical Review Focusing on Comorbidities." *ACS chemical neuroscience* vol. 9,2 (2018): 167-176. doi:10.1021/acschemneuro.7b00346. [2]Armstrong, Dawna Duncan. "Neuropathology of Rett syndrome." *Journal of child neurology* vol. 20,9 (2005): 747-53. doi:10.1177/08830738050200090901.

There are no approved disease-modifying treatments that address the genetic root cause of Rett syndrome

High Unmet Medical Need

- Current standard of care focused on symptom management[1]
- Patients typically require 24/7 care and lifelong assistance[2]
- High caregiver burden with significant impact on quality of life and activities of daily living[2]

Significant Market Opportunity

- Estimated **15,000 and 20,000 patients in major global markets** (U.S., EU+U.K.)[3]
- **1 of every 10,000 female births worldwide**[3]
- Commercial launch and uptake of DAYBUE highlights market demand[4]

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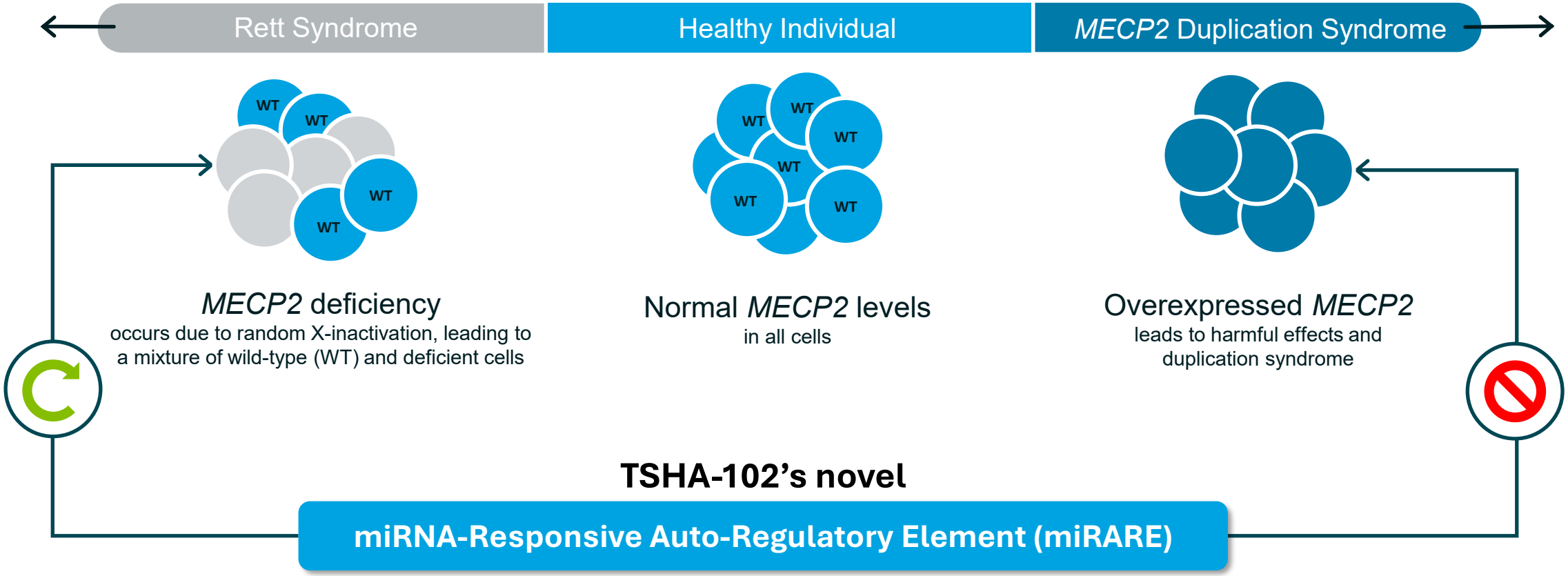
[1]Fu, Cary et al. "Consensus guidelines on managing Rett syndrome across the lifespan." *BMJ paediatrics* open vol. 4,1 e000717. 13 Sep. 2020, doi:10.1136/bmjpo-2020-000717. [2]Coenraads, Monica et al. "Voice of the Patient Report: *Rett Syndrome Externally-Led Patient-Focused Drug Development Meeting*." 9 Aug. 2022, https://rettpfdd.org/site/assets/files/1/2022-rett-syndrome-voice-of-the-patient-report.pdf. [3]Amir, R E et al. "Rett syndrome is caused by mutations in X-linked MECP2, encoding methyl-CpG-binding protein 2." *Nature genetics* vol. 23,2 (1999): 185-8. doi:10.1038/13810. (estimated prevalence of *15,000-20,000 patients with typical Rett syndrome caused by a MECP2 mutation*).[4]Based on net product sales for the three months ended March 31, 2025 (source: Acadia Pharmaceuticals Reports First Quarter 2025 Financial Results and Operating Overview).

Gene Therapy Treatment Challenge: **too much or too little *MECP2* expression is harmful in Rett syndrome**



technology is designed to correct *MECP2* deficiency and avoid toxic overexpression

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[1]Van Esch, Hilde et al. "Duplication of the MECP2 region is a frequent cause of severe mental retardation and progressive neurological symptoms in males." *American journal of human genetics* vol. 77,3 (2005): 442-53. doi:10.1086/444549.

TSHA-102: an investigational one-time gene therapy that is designed to regulate *MECP2*

Strategically designed to enable optimal and controlled transgene expression across the CNS

AAV9 Capsid

Clinically and commercially proven CNS capsid

Self-complementary Vector

Enables more rapid, potent and efficient transgene expression vs. traditional single-stranded viral genome[1]

***miniMECP2* Transgene**

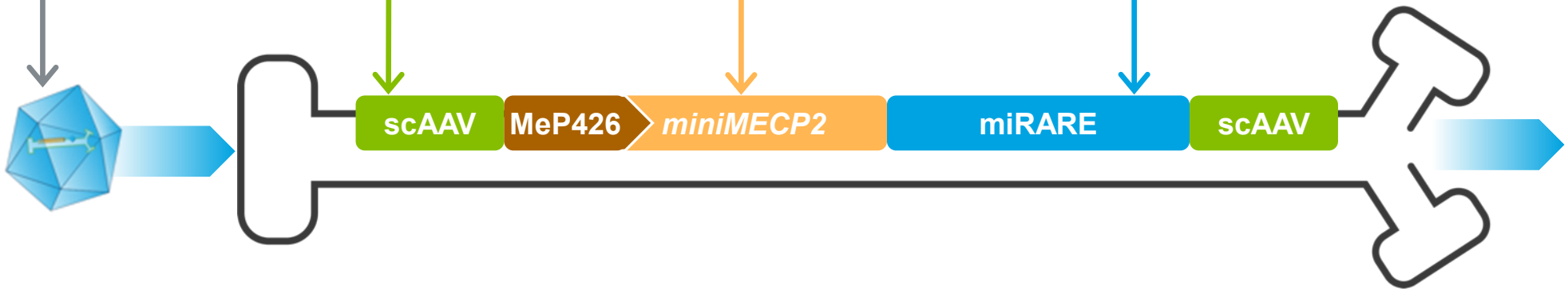
Contains essential functional domains, and smaller size enables packaging of construct in self-complementary viral genome[2]

miRARE Technology

Regulates transgene expression on a cell-by-cell basis to address risks associated with under- and over-expression by sensing both transgene and endogenous *MECP2* levels[3]

Intrathecal (IT) Delivery

Leads to widespread and consistent biodistribution across the brain and spinal cord using a routine, minimally invasive procedure with potential for outpatient administration[4]



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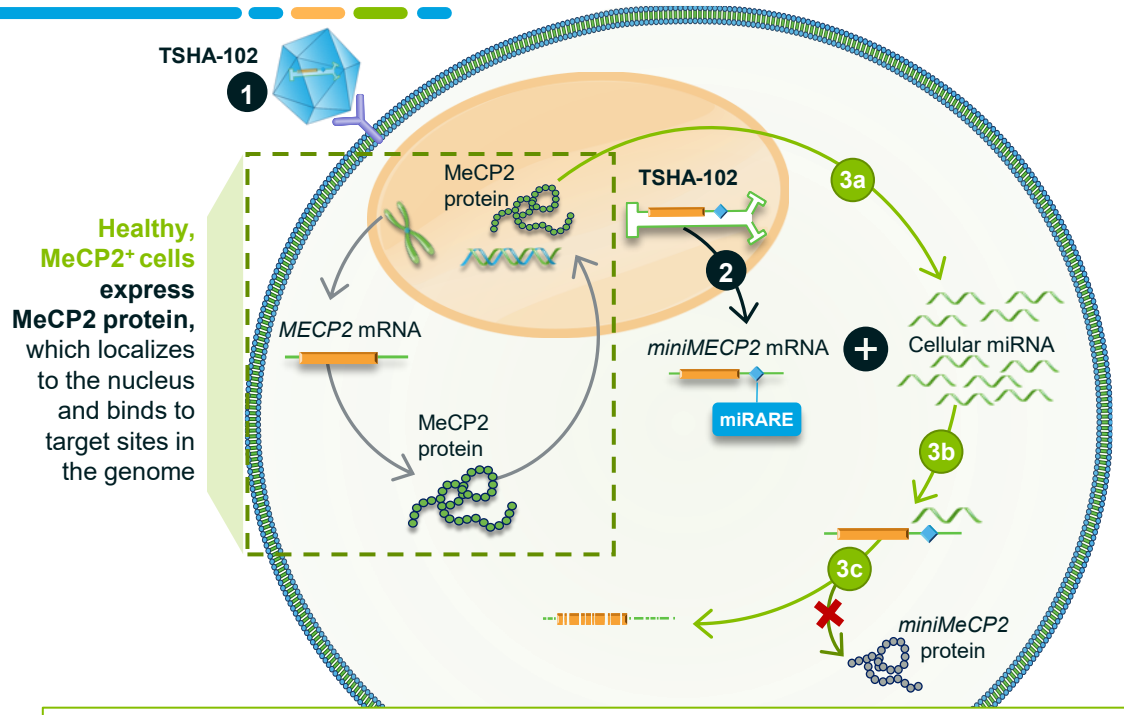
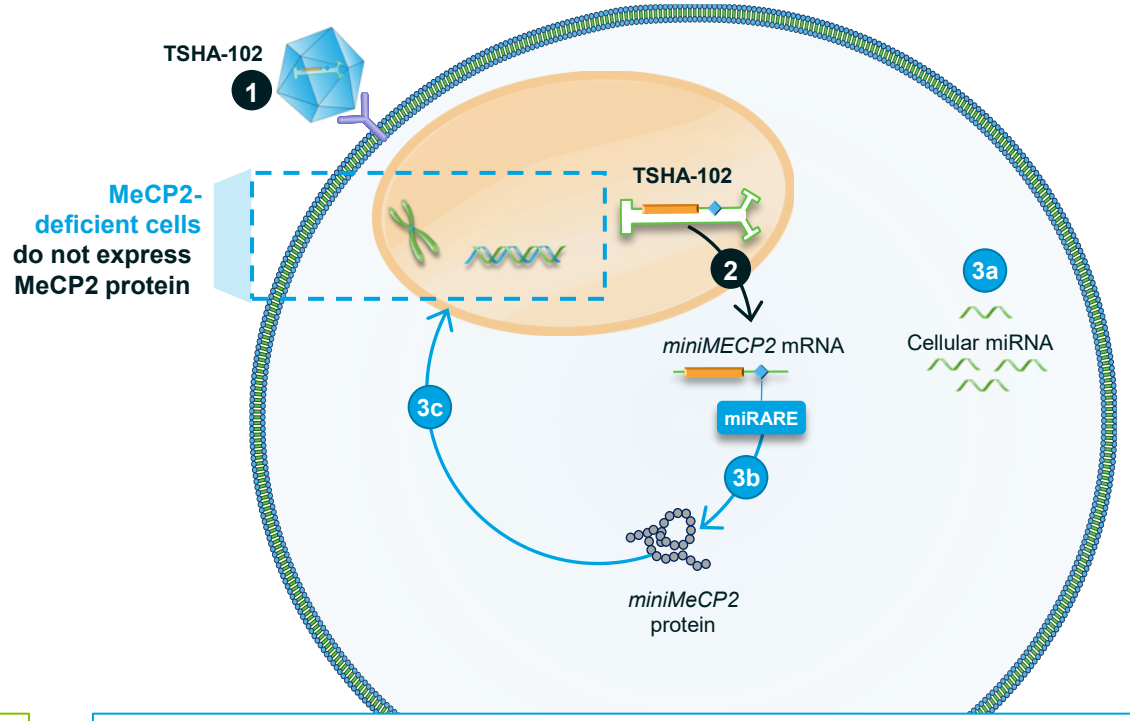
[1]Gray, Steven J et al. "Preclinical differences of intravascular AAV9 delivery to neurons and glia: a comparative study of adult mice and nonhuman primates." Molecular therapy : *the journal of the American Society of Gene Therapy* vol. 19,6 (2011): 1058-69. doi:10.1038/mt.2011.72. [2]Tillotson, Rebekah et al. "Radically truncated MeCP2 rescues Rett syndrome-like neurological defects." *Nature* vol. 550,7676 (2017): 398-401. doi:10.1038/nature24058. [3]Haque, Emdadul et al. "The microRNA-responsive autoregulatory element from TSHA-102 for Rett Syndrome modulates therapeutic transgene expression in response to cellular MeCP2 in mouse and human cell lines." 30th Annual Congress of the European Society for Gene and Cell Therapy, 25-26 Oct. 2023, Brussels, Belgium. [4]Haque et al. "rAAV9 vector biodistribution in nonhuman primate brain and spinal cord following lumbar intrathecal infusion" *Front. Med.* 13:1819594. doi: 10.3389/fmed.2026.1819594

miRARE: potential best-in-class approach to regulating *MECP2* expression using RNA interference and binding sites for endogenous microRNA responsive to MeCP2[1]





miRARE is designed to SILENCE *miniMECP2* transgene expression in healthy, MeCP2⁺ cells

In cells with normal MeCP2 function in the nucleus:

3a	3b	3c
Cellular miRNAs are produced abundantly	miRNAs interact with miRARE in the mini*MECP2* mRNA	This interaction signals the cell to degrade the mRNA and/or to **suppress synthesis of the miniMeCP2 protein**

miRARE is designed to ENABLE *miniMECP2* transgene expression in MeCP2-deficient cells

In cells lacking normal MeCP2 function:

3a	3b	3c
Fewer cellular miRNAs are produced	Therefore, the transgene mRNA is translated to produce miniMeCP2 protein	The miniMeCP2 protein is imported into the nucleus, **restoring MeCP2 function**

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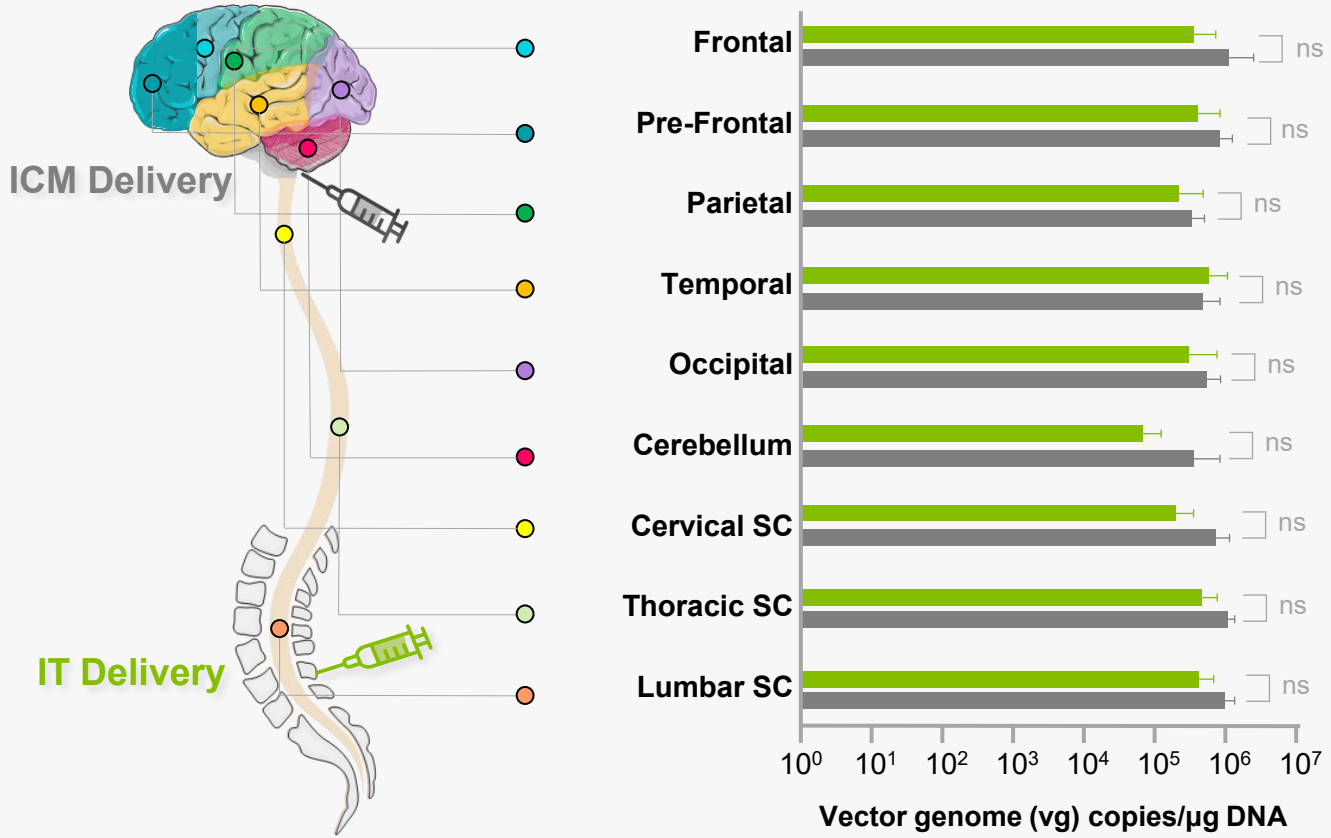
[1]Sinnett, Sarah E et al. "Engineered microRNA-based regulatory element permits safe high-dose miniMECP2 gene therapy in Rett mice." *Brain*: a journal of neurology vol. 144,10 (2021): 3005-3019. doi:10.1093/brain/awab182.

Intrathecal (IT) Administration: a potentially effective, safe and minimally invasive delivery approach for broad targeting of the CNS[1]

- IT and ICM administration achieved **comparable, consistent and broad biodistribution** in the brain and spinal cord
- Supports TSHA-102 clinical development approach **leveraging the minimally invasive IT administration** with potential for **outpatient administration**
- IT administration **minimizes systemic exposure**, potentially reducing risk of immune responses and systemic toxicities

Biodistribution data comparing IT vs. ICM delivery using the same dose and volume in NHPs[1]



TSHA-102 (ICM): $2x10^{15}$ total vg HED, TSHA-105 (IT): $2x10^{15}$ total vg HED

[1]Haque et al. "rAAV9 vector biodistribution in nonhuman primate brain and spinal cord following lumbar intrathecal infusion" *Front. Med.* 13:1819594. doi:10.3389/fmed.2026.1819594

ICM=Intra-cisterna magna; NHP=Non-human primate; HED=Human equivalent dose; SC=Spinal cord; ns=Not significant

FDA aligned pathway to potential registration for TSHA-102

PART A: REVEAL Phase 1/2 Trials

Adolescent and Adult (females ≥12 years)

Cohort 1
5.7x10^{14} total vg
N=2

→ Cohort 2
1x10^{15} total vg
N=4

Pediatric (females 5-8 years)

Cohort 1
5.7x10^{14} total vg
N=2

→ Cohort 2
1x10^{15} total vg
N=4

Dosing complete (N=12 females, 6-21 years)

PART B: REVEAL Pivotal Trial

Developmental Plateau Population
(females 6 to <22 years)

- Evaluate efficacy and safety of TSHA-102

N=15
1x10^{15} total vg

ASPIRE Trial

Pre-developmental Plateau Population
(females 2 to <4 years)

- Evaluate safety and preliminary efficacy of TSHA-102; efficacy data to be extrapolated from REVEAL pivotal trial

N=3
1x10^{15} total vg[1]

Potential Registrational Path

Patients with Rett syndrome Age 2+

Written FDA alignment on:

- REVEAL 6-month interim analysis may serve as basis for BLA submission
- Inclusion of ≥3 months of ASPIRE safety data in BLA submission to support a broad label in patients aged ≥2 years

[1]*The 1x10^{15} total vg* dose administered in ASPIRE will be scaled to account for the lower brain volume in 2 to <4-year-olds

Ongoing REVEAL Pivotal Trial for TSHA-102

Finalized FDA alignment on study protocol and SAP

Study Overview

- **Study Design:** Single-arm, open-label trial, using each patient as own control
- **Dose:** 1x10^{15} total vg (high dose), administered intrathecally
- **Sample Size:** Enrolling 15 females with Rett syndrome aged 6 to <22 years (developmental plateau population)[1]
- **Primary Endpoint:** Response rate, defined as the percentage of patients who gain or regain ≥ one developmental milestone from a list of 28
  - Video-based determination of milestone gain/regain will be performed by independent, blinded central raters
- **SAP:** Response rate of 33% (5 out of 15 patients) is the minimum threshold for success sufficient to reject the null hypothesis of 6.7%[2]
  - 12-month primary analysis, with 6-month interim analysis that may serve as the basis for BLA submission
- **Key Secondary Endpoints:** Average number of total developmental milestones gained/regained per patient and clinician-assessed outcome measures, including R-MBA and CGI-I

Company Believes REVEAL Part A Data Support Pivotal Trial is Well Powered to Establish Efficacy

Response rate = 100% (N = 10) across all patients treated with TSHA-102 post-treatment[3]

[1]Patients aged ≥6 years have a 0% to <6.7% likelihood of gaining new or regaining developmental milestones that were lost after a defined number of years, based on Taysha's natural history data analysis (N = ~1100 females with Rett syndrome, with up to 14 years follow-up). [2]Based on Taysha's natural history data analysis, the null hypothesis is that one out of 15 patients aged ≥6 years may gain/regain one of the 28 natural history defined developmental milestone without treatment, corresponding to a response rate of 6.7%. [3]Based on May 19, 2025, data cutoff. Study is ongoing, and data is subject to change.
SAP=Statistical analysis plan; BLA=Biologics license application; R-MBA=Revised Motor Behavior Assessment; CGI-I=Clinician Global Impression–Improvement

Primary endpoint: milestone gain is an objective, clinically meaningful and inherently individualized assessment of function in the developmental plateau population

~0% probability of milestone gain/regain after age 6 in the untreated population[1]

Supported by cumulative incidence models from longitudinal natural history study developmental milestone data

Fine motor, gross motor, and communication developmental milestones captured as binary (yes/no) measures

Gain or regain of ≥ one of 28 defined developmental milestones post-TSHA-102

- ✓ Represent meaningful functional improvement based on caregiver research[2]
- ✓ Directly reflects activities of daily living
- ✓ Inherently individualized to show improvements in a heterogeneous disease
- ✓ FDA-endorsed primary endpoint

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[1]Incidence models derived from NHS data; accessed from IRSF. ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT. Cumulative incidence models of NHS data conducted by third-party statistical partners. [2]Downs, Jenny. "Every Gain, Expanding Possibilities: Caregiver Insights on Meaningful Improvement in Rett Syndrome Gene Therapy (GT)". 2025 IRSF Rett Syndrome Scientific Meeting, 9-11 June 2025, Boston.
NHS=Natural history study

28 developmental milestones from the natural history dataset included in the primary endpoint

Communication

- Pointed for something they wanted
- Waved “Bye-Bye”
- Followed a command with a gesture
- Identified body parts (pointed with eyes or fingers)
- Followed a command without a gesture
- Used word(s) with meaning
- Spoke in phrases (2 words or more) with meaning

Fine Motor

- Reached for toy
- Holds bottle unpropped
- Used raking grasp to retrieve an object
- Used pincer grasp (refined or modified)
- Transferred an object from one hand to another
- Finger fed
- Drank from a cup held without assistance
- Used a fork or spoon to eat with assistance
- Used a fork or spoon to eat without assistance

Gross Motor

- Come to sitting
- Sat without support
- Stood while holding on
- Pulled to standing
- Stood independently
- Walked with support
- Walked independently
- Climbed up stairs with help
- Climbed down stairs with help
- Climbed up stairs without help
- Climbed down stairs without help
- Ran 10 feet without falling

Selection criteria:

- ✓ **Meaningful functional gains based on caregiver research**
- ✓ **Represent activities of daily living**
- ✓ **0% to <6.7% likelihood gained/regained in patients age ≥ 6 years with untreated Rett syndrome[1]**

[1]Accessed from IRSF. ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT. Cumulative incidence models of NHS data conducted by third-party statistical partners.
NHS=Natural history study

Key findings that supported alignment with FDA on the REVEAL pivotal trial

1

Rett Syndrome Caregiver Research

achievement of a developmental milestone would be a meaningful therapeutic outcome

2

Rett Syndrome Natural History Data Analysis

by age ≥ 6 years, there is ~0% likelihood of gaining or regaining developmental milestones[1]

3

Part A Clinical Data

100% of patients gained/regained ≥ one developmental milestone post-TSHA-102[2]

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[1]Accessed from IRSF. ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT. Cumulative incidence models of NHS data conducted by third-party statistical partners. [2]Efficacy data based on May 19, 2025, data cutoff (N=10); Study is ongoing and data is subject to change.

Rett syndrome caregiver research indicates improved function or achievement of developmental milestones would significantly improve quality of life

Communication

Gained or improved communication of basic needs—through eye gaze, gestures, or words—would enable self-advocacy and strengthen social connections

- Ex: follow a command without a gesture, pointed for something they wanted, use word(s) with meaning, identify body parts (pointed with eyes or fingers)

Fine Motor Function

Gained or improved hand function would restore a sense of control and purpose, and enable play and social engagement

- Ex: finger feed, use fork or spoon to eat without assistance, reached for a toy, drank from a cup held without assistance

Gross Motor Function

Gained or improved gross motor function would foster independence and reduce the physical burden of caregiving

- Ex: walked independently or with support, stood while holding on, sat without support, climbed up stairs without help

"If she can actually tell me what she wants, or make a choice between two things, even if it's just looking at something purposefully…because now I don't know what's going on."
– Caregiver of 20-year-old

"Feeding herself, entertaining herself…being able to flip pages or purposefully hold a book, change the channel on a remote...would be a game changer for us."
– Caregiver of 8-year-old

"If we got a safe and secure sitting position from her, that would be a win. We would be able to have her sitting and not have to be right next to her. We could have her at the dining table with us."
– Caregiver of 5-year-old

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Keeley, Jessica et al. "Every Gain, Expanding Possibilities: Caregiver Insights on Meaningful Improvement in Rett Syndrome Gene Therapy." 2025 IRSF Rett Syndrome Scientific Meeting, 9-11 June 2025, Boston. Poster Presentation.

Longitudinal natural history data informed key elements of TSHA-102 pivotal trial design

Natural History Study (NHS) Dataset[1]

- N = ~1100 females with confirmed Rett syndrome diagnosis; up to 14 years follow-up
- Captures longitudinal data on the functional gain, loss and regain of **developmental milestones** across core domains of Rett syndrome:

Communication
Ex: Pointed for something they wanted | Used word(s) with meaning

Fine Motor Function
Ex: Finger feed | Drank from a cup held without assistance

Gross Motor Function
Ex: Sat without support | Walked with support

- These functional skills and activities of daily living are highly important to caregivers

Developed Age- and Time-Based Models of Developmental Milestone NHS Data[1]

- Cumulative incidence models demonstrated **distinct trends in developmental milestone acquisition** that:

✓ Strengthened understanding of longitudinal disease progression in Rett syndrome

✓ Contextualized and substantiated disease-modifying potential of TSHA-102

✓ Informed our discussions with the FDA on proposed pivotal trial design for TSHA-102

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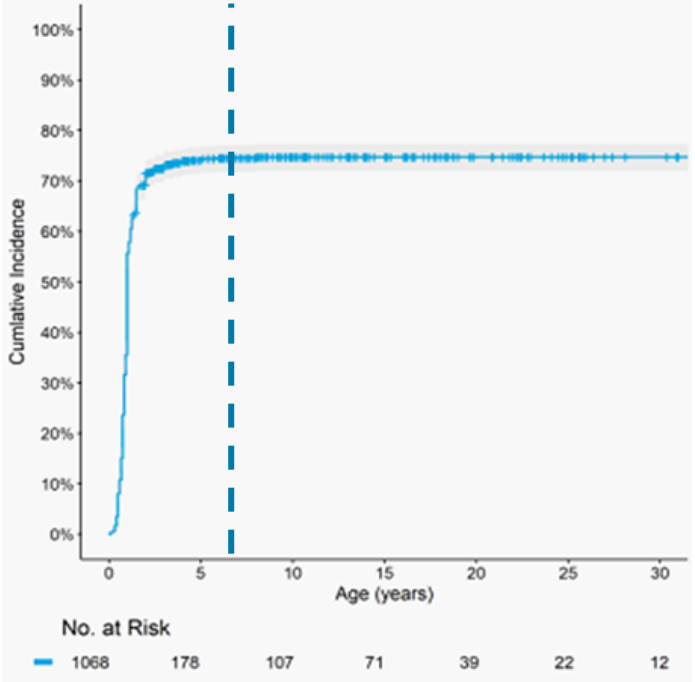
[1]Accessed from IRSF. ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT. Cumulative incidence models of NHS data conducted by third-party statistical partners.

NHS cumulative incidence models showed that the likelihood of gaining/regaining 28 defined developmental milestones is **predictable in the age ≥ 6 population**[1]

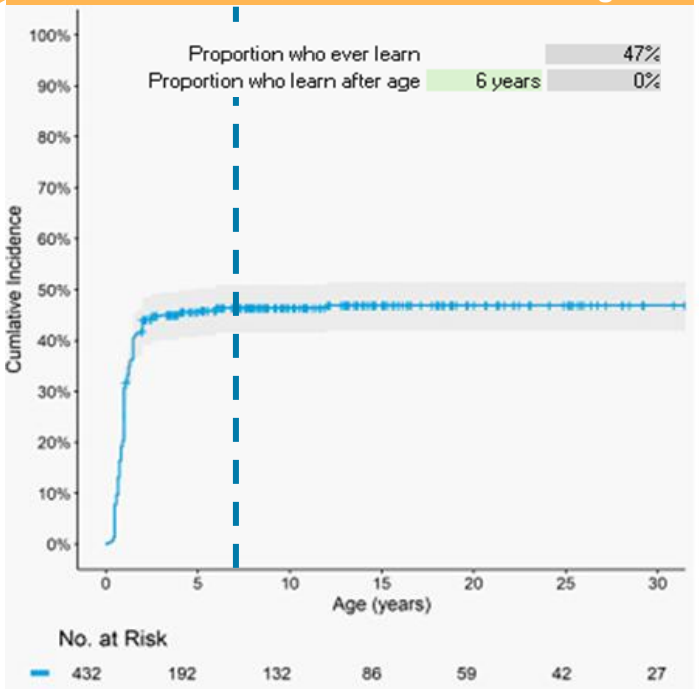
Communication Milestones

Ex: of patients who learn to use word(s) with meaning, **~0% ever learn after age 6**



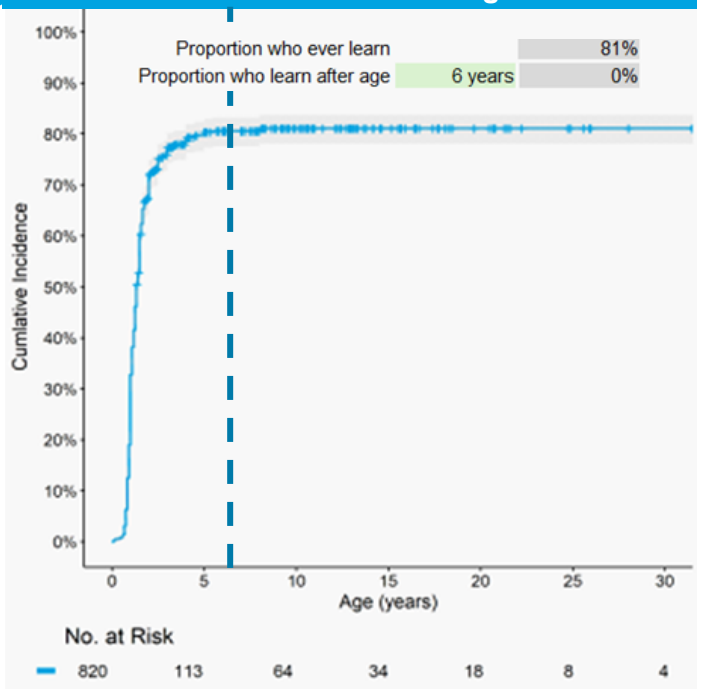
Fine Motor Milestones

Ex: of patients who learn to drink from a cup held without assistance, **~0% ever learn after age 6**



Gross Motor Milestones

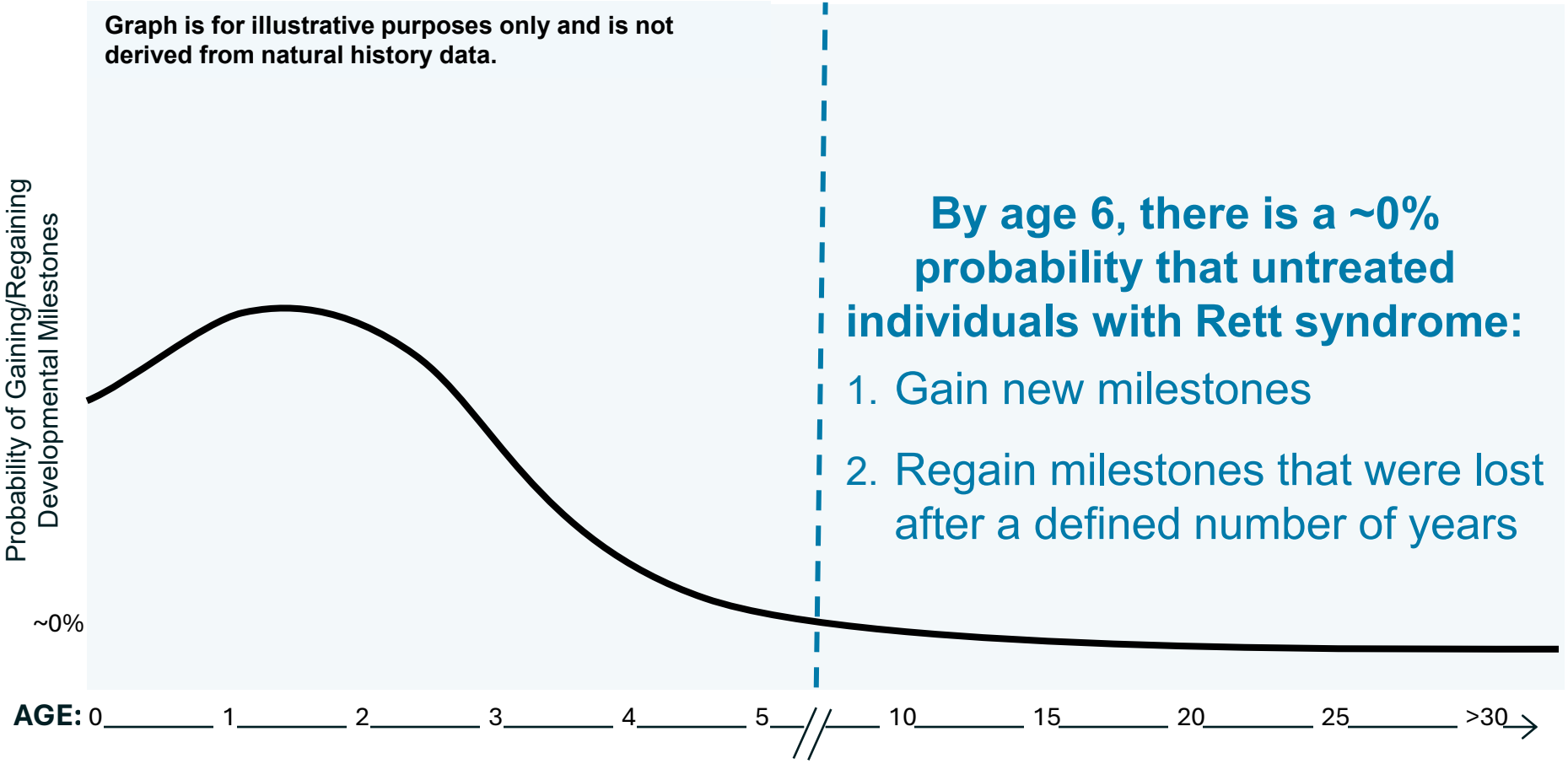
Ex: of patients who learn to walk with support, **~0% ever learn after age 6**



We leveraged these findings to establish the **"Developmental Plateau Population"**

[1]Accessed from IRSF. ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT. Incidence models of NHS data conducted by third-party statistical partners. NHS=Natural history study

Rett syndrome NHS data analysis demonstrated that **after 6 years of age, there is ~0% likelihood of gaining or regaining developmental milestones**[1]



Pre-Developmental Plateau Population (age 0-6 years): demonstrate ongoing development

Developmental Plateau Population (age ≥6 years): gain or regain of developmental milestones is unexpected

NHS data analysis supports a minimum inclusion age of 6 years in a well-controlled, single-arm interventional trial evaluating gain and regain of developmental milestones

[1]Accessed from IRSF. ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT. Cumulative incidence models of NHS data conducted by third-party statistical partners.
NHS=Natural history study

REVEAL Part A data supports encouraging safety profile and disease-modifying potential of TSHA-102[1]

Underscores the potential to improve function and enable developmental milestone achievements

Generally Well-tolerated	no treatment-related SAEs or DLTs in all patients treated to date
Developmental Milestone Achievement	in 100% of patients, which is not expected based on natural history, with a pattern of early sustained gains and new gains/regains over time
Additional Skills and Improvements	in 100% of patients, with multiple skill gains/improvements outside the natural history defined developmental milestones across the core domains of Rett syndrome
Dose-dependent Improvements	high dose consistently outperforming low dose, and effects deepening over time across measures (e.g., developmental milestones, R-MBA, CGI-I)

[1]Efficacy data based on May 19, 2025, data cutoff (N=10); Safety data based on May 2026 data cutoff (N=12). Study is ongoing and data is subject to change.
SAE=Serious adverse event; DLT=Dose-limiting toxicity; R-MBA=Revised Motor Behavior Assessment; CGI-I=Clinical Global Impression-Improvement

Part A of REVEAL Phase 1/2 trials of TSHA-102

Dosing complete (N=12, aged 6-21 years)

Study overview:

- Open-label, dose-escalation and dose-expansion trial in the U.S. and Canada
- Safety and preliminary efficacy of TSHA-102
- Evaluates two dose levels; if possible, establishes MAD or MTD

Key clinical assessments:

- R-MBA
- CGI-I
- CGI-S

Adolescent and Adult Trial

Females aged ≥12 years

Low dose

Cohort 1
$5.7x10^{14}$ total vg

Completed (N=2)

High dose

Cohort 2
$1x10^{15}$ total vg

Completed (N=4)

Pediatric Trial

Females aged 5-8 years

Low dose

Cohort 1
$5.7x10^{14}$ total vg

Completed (N=2)

High dose

Cohort 2
$1x10^{15}$ total vg

Completed (N=4)

MAD=Maximum administered dose; MTD=Maximum tolerated dose; vg=Vector genomes; R-MBA=Revised Motor Behavior Assessment Scale; CGI-I= Clinical Global Impression Scale-Improvement; CGI-S=Clinical Global Impression Scale-Severity

Rigorous developmental milestone evaluation criteria applied to Part A data enabled reliable, objective assessment of TSHA-102 efficacy

Developmental milestones data was captured via videos, COAs, caregiver assessments and clinician notes in Part A

Developmental milestone evaluation criteria:

- ✓ **Baseline:** Milestone either never gained or lost sufficiently long ago, such that the likelihood of spontaneous gain/regain is <6.7%, based on review of patient medical history and available baseline video data
- ✓ **Post-treatment evidence:** Video documentation of milestone demonstration
- ✓ **Evaluation method:** Milestone gain/regain determined by multiple independent central raters based on prespecified definitions of achievement for each milestone outlined in the pivotal trial protocol

Reliable Part A data supporting TSHA-102's impact on developmental milestone achievement indicate the pivotal trial is well powered to establish efficacy of TSHA-102

COAs=Clinical outcome assessments

100% of patients (N=10) gained/regained ≥ one defined developmental milestone post-TSHA-102

with a **~0% likelihood** of being achieved without treatment based on NHS data[1]

	Cohort 1: Low Dose 5.7x10^{14} total vg				Cohort 2: High Dose 1x10^{15} total vg					
	LD:P1	LD:P2	LD:P3	LD:P4	HD:P1	HD:P2	HD:P3	HD:P4	HD:P5	HD:P6
AGE AT DOSING (years):	20	21	6	7	15	21	16	8	6	7
POST-TREATMENT FOLLOW UP:	18 mos.	18 mos.	12 mos.	12 mos.	9 mos.	9 mos.	6 mos.	6 mos.	6 mos.	3 mos.
	Developmental Milestone Gained Post-TSHA-102				Developmental Milestone Gained Post-TSHA-102					
	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓

Developmental milestone gains and regains were assessed by multiple independent central raters, who evaluated functional skills through video evidence at baseline and post-treatment, applying predefined binary criteria.

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Based on May 19, 2025, data cutoff (N=10). Data presented reflects current data in the Electronic Data Capture System, subject to change. [1]Incidence models derived from NHS data; accessed from IRSF. ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT. Cumulative incidence models of NHS data conducted by third-party statistical partners.
NHS=Natural history study; vg=Vector genomes

Patients gained/regained developmental milestones **across the core functional domains** of Rett syndrome post-TSHA-102

A total of 22 developmental milestones were achieved across 10 patients treated with TSHA-102

Communication

- Spoke in phrases (2 words or more) with meaning
- Used word(s) with meaning
- Followed a command without a gesture
- Followed a command with a gesture
- Pointed for something they wanted
- Identified body parts

Enable **expression of needs**, preferences, emotions, and foster **social connections**

Fine Motor

- Holds bottle unpropped
- Finger fed
- Reached for a toy
- Transferred an object from one hand to another

Reflect self-care skills and purposeful hand use that **enable independence**

Gross Motor

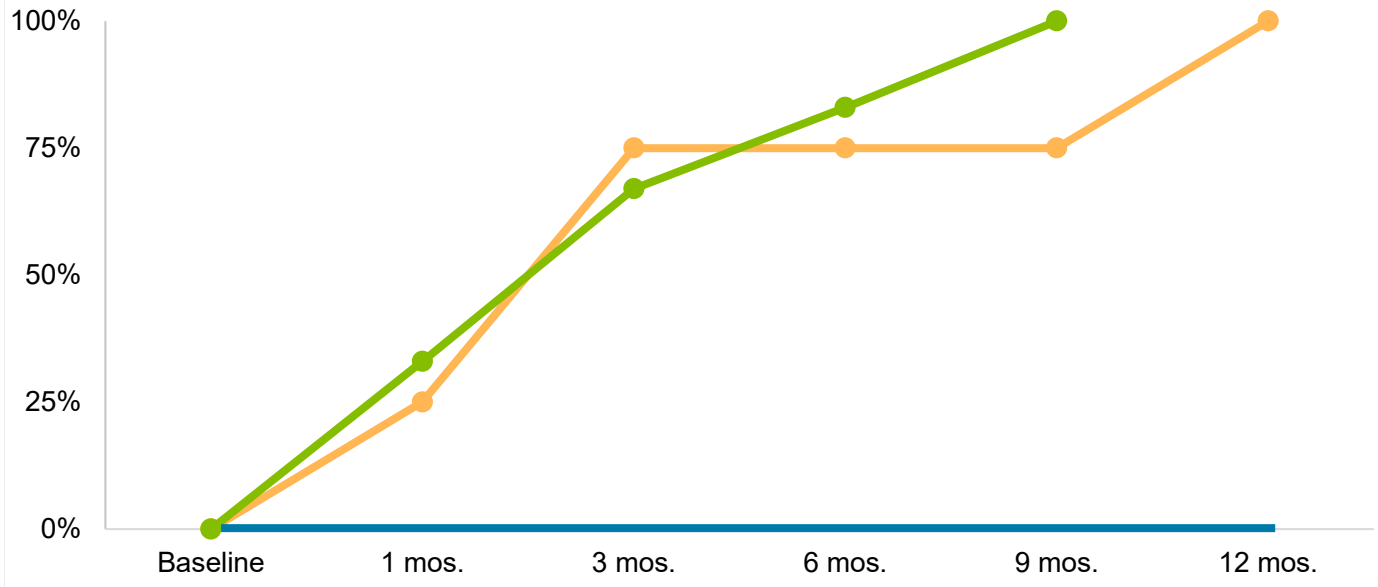
- Walked with support
- Stood while holding on
- Pulled to standing
- Sat without support

Enhance mobility and independence, and **reduce the physical burden of caregiving**

Based on May 19, 2025, data cutoff (N=10). Developmental milestone gains and regains were assessed by multiple independent central raters through video evidence.

High dose TSHA-102 achieved 100% responder result at a **25% faster rate** compared to low dose TSHA-102

Responder Rate: Time to Response



	Baseline	1 mos.	3 mos.	6 mos.	9 mos.	12 mos.
Low Dose TSHA-102	25%	75%	75%	75%	100%	
High Dose TSHA-102	33%	67%	83%	100%		

Natural History (Developmental Plateau Population)[1] = ~0%

- **Accelerated functional benefit seen with high dose TSHA-102**
- Early clinical response may increase the likelihood of reversing the disease trajectory and may be predictive of long-term clinical outcomes in Rett syndrome
- Consistent pattern of early gains that are sustained, with new achievements continuing to emerge over time following TSHA-102

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Based on May 19, 2025, data cutoff (N=10). Data presented reflects current data in the Electronic Data Capture System, subject to change. Low Dose N=4 across all time points; High Dose N=6 at 1-3 months., N=5 at 6 months, N=2 at 9 months, based on available follow up. [1]Accessed from IRSF. ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT.Cumulative incidence models of NHS data conducted by third-party statistical partners.

REVEAL caregiver testimonials post-TSHA-102 highlight the impact of functional **developmental milestone gains** on quality of life

"All of our days are better. Her improvements are much beyond anything we had expected or hoped for."

"She's **gained multiple words** – 'no,' 'yeah,' 'mom,' 'dad' – makes consistent sounds with meaning – and even **says some phrases** – 'ok, bye' and 'no more.'"

"She's a lot easier to care for. She can **point a lot more deliberately to make choices and show us what she wants**, and she will keep gesturing until we get it for her. And she pushes away what she doesn't want."

"[**Standing while holding on**] has been a godsend when it comes to toileting while out in the community because now, I can have her stand and hang on to my arm to toilet or wipe her… and the consistency of keeping her hand down [without constant stereotypies] allows us to practice more with a walker, which has been huge."

"Her hands are more relaxed, and she tries to grab everything with a **raking grasp**.
She can **follow directions** in a snap, like if we say, 'let's go,' she gets up, heads to the door.
She's babbling now, which she didn't do before, and is definitely trying to tell us something."

Based on documented testimonials from caregivers of participants treated with TSHA-102 in REVEAL Phase 1/2 trials, as of May 19, 2025, data cutoff.

Systematic evaluation criteria in Part A demonstrated a broad functional impact of TSHA-102

Primary Evidence of Efficacy

Developmental Milestones (DM)

Functional gain of ≥ one of the **28 DMs defined in the natural history study** assessed via rigorous video-evidenced evaluation

Evaluation Criteria:

- ✓ **Baseline:** Video data/medical history confirming milestone was either never gained or lost sufficiently long ago, such that the likelihood of spontaneous gain/regain is <6.7%[1]
- ✓ **Post-treatment:** Video documentation of milestone demonstration
- ✓ **Evaluation method:** Determined by multiple independent central raters based on prespecified definitions of achievement for each milestone

Supportive Evidence of Functional Gain

Additional Skills and Improvements

Functional gain or improvement in a core disease characteristic **outside of the 28 natural history defined DMs** measured by validated, structured scales

Rett-validated Scale:	Purpose:	Data Collection and Evaluation Method:
Adapted Mullen Scales of Early Learning (MSEL-A)	Assesses expressive and receptive language skills	✓ Video-recorded evaluation conducted in clinic ✓ Centrally rated by multiple independent raters ✓ N=5 patients with available data
Revised Motor Behavior Assessment (R-MBA)	Assesses frequency, severity or independence of Rett characteristics	✓ Video-recorded evaluation conducted in clinic ✓ Clinician-reported ✓ N=10 patients with available data
Observer-Reported Communication Ability (ORCA)	Assesses communication skills	✓ Structured evaluation conducted in home ✓ Caregiver-reported ✓ N=5 patients with available data

All reported skills/improvements required: **(a)** baseline data, **(b)** documented gain or improvement for individual item that was sustained through latest assessment

[1]Accessed from IRSF. ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT. Incidence models of NHS data conducted by third-party statistical partners.

100% of patients (N=10) achieved multiple skills/improvements outside the natural history defined developmental milestones post-TSHA-102

Reinforce broad, consistent functional gains across the core domains impacting activities of daily living

A Total of 165 Additional Skills and Improvements Achieved Across Core Disease Characteristics[1]

MSEL-A = 34 total gains

- Played with sounds
- Vocalized two-syllable sounds
- Recognized body parts (one or more)
- Identified objects
- Understood action words
- Identified the function of objects
- Identified colors (one or more)
- Understood size/length concepts
- Understood comparative concepts
- Followed directions
- Followed two related commands
- Followed three unrelated commands
- Differentiated between objects with increasing levels of abstraction and complexity

ORCA = 59 total gains

- Used gesture to communicate (e.g. point, eye gaze)
- Understood mood (based on facial expression/tone of voice)
- Anticipated a game/activity in response to verbal cue
- Responded when told “no” or “stop”
- Expressed refusal by saying "no”
- Used gesture to say “hello” and “goodbye”
- Used gesture/word to identify a person by name
- Responded to yes/no and open-ended questions
- Took turns during game/activity
- Used AAC device to communicate
- Followed two-step directions part of daily routine
- Expressed frustration when misunderstood
- Communication understood by known individuals

R-MBA = 72 total improvements

Improved:

- Motor skills
- Purposeful hand use
- Frequency of reach for objects/people
- Chewing function
- Speech and verbal communication
- Response to spoken words
- Ability to make choices
- Sustained social interest and eye contact

Reduced or Absent:

- Hand stereotypies
- Breath holding and hyperventilation
- Seizure episodes
- Choking/gagging episodes
- Dystonia and hypertonia/rigidity
- Bradykinesia
- Truncal rocking
- Bruxism
- Aberrant behavior (e.g. self harming)

Based on May 19, 2025, data cutoff (N=10). Data presented reflects current data in the Electronic Data Capture System, subject to change. [1]Rossignol E, et al. "Achieving Developmental Milestones and Broad Restoration of Function in Rett Syndrome: The Potential of TSHA-102 Gene Therapy.” 54th Child Neurology Society Annual Meeting, 8-11 October 2025, Charlotte. Poster Presentation.
AAC=Augmentative and Alternative Communication device.

Examples of multi-domain functional gains and improvements impacting activities of daily living observed post-TSHA-102

Based on developmental milestone gains and additional skills/improvements among the 10 treated patients[1]

Communication Improvements

Enable **expression of needs**, preferences, emotions, and foster **social connections**

PRE-TREATMENT	POST-TREATMENT
Non-verbal	Uses phrases/ sentences with meaning
Understood simple words	Complex ideas and engaging in conversations
Made choices <10% of time using eye gaze	Makes choices 100% of time by pointing

Fine Motor Improvements

Reflect self-care skills and purposeful hand use that **enable independence**

PRE-TREATMENT	POST-TREATMENT
Stereotypies 76-100% of time	Stereotypies 1-25% of time
No purposeful hand use	Plays with toys and transfers intentionally
Required caregiver-assisted feeding	Finger feeds and holds a bottle unpropped

Gross Motor Improvements

Enhance mobility and independence, and **reduce the physical burden of caregiving**

PRE-TREATMENT	POST-TREATMENT
Most severe dystonia (fixed positional deformity)	No dystonia
Non-ambulatory	Walks with support
Required caregiver support for positional transfers and to stand	Pulls self to standing position and stands while holding on

Autonomic / Other Improvements

Improve core features of Rett syndrome that have a **meaningful impact on quality of life**

PRE-TREATMENT	POST-TREATMENT
Hyperventilating/ breath holding 26-50% of time	Absent or reduced hyperventilating/ breath holding
Weekly-monthly seizure episodes	Seizure-free ≥6 months
Required a g-tube for feeding	Oral feeds

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Based on May 19, 2025, data cutoff (N=10). Data presented reflects current data in the Electronic Data Capture System, subject to change. [1]Rossignol E, et al. "Achieving Developmental Milestones and Broad Restoration of Function in Rett Syndrome: The Potential of TSHA-102 Gene Therapy." 54th Child Neurology Society Annual Meeting, 8-11 October 2025, Charlotte. Poster Presentation.

TSHA-102 demonstrated a **statistically significant** mean R-MBA score improvement compared to natural history at both 6 and 12 months

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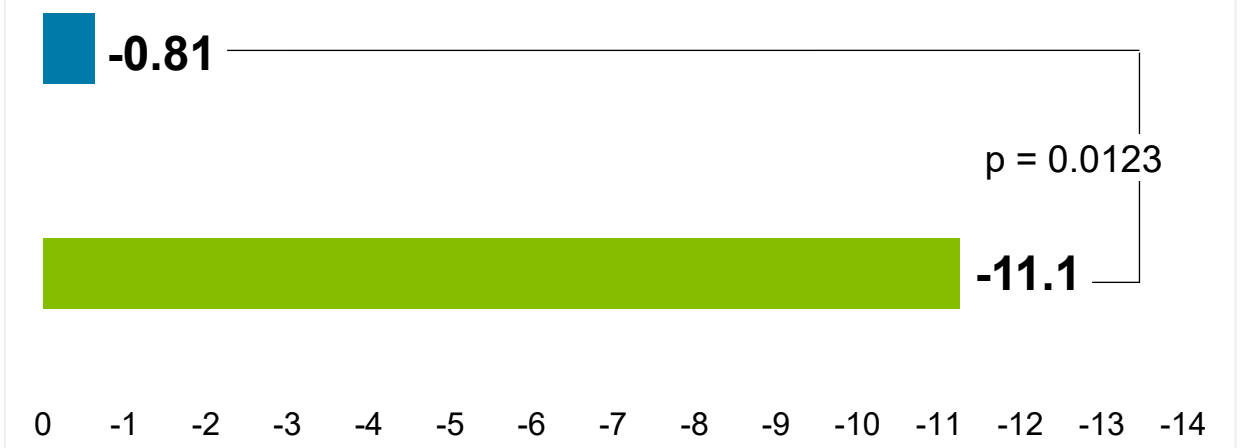
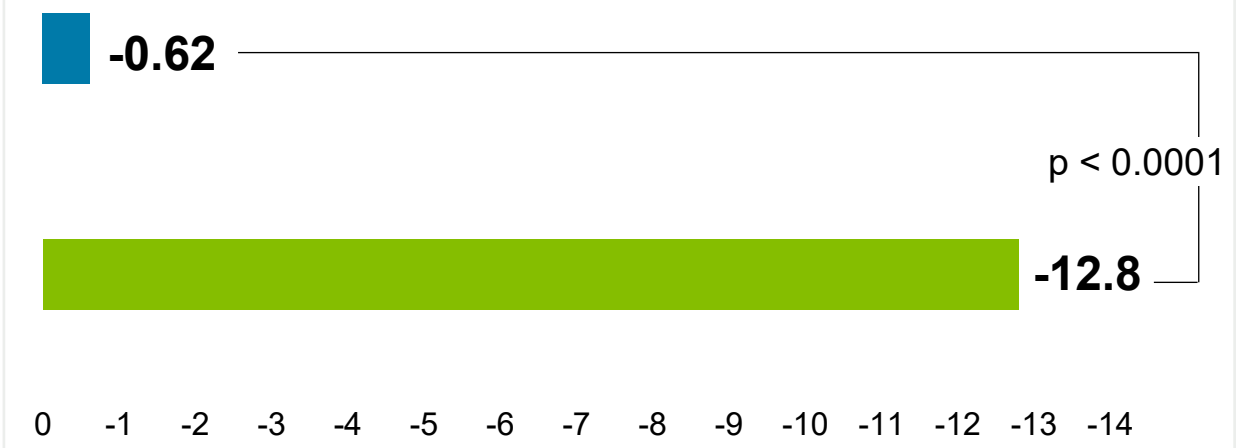
Lower score = improvement; R-MBA assessed in Rett syndrome NHS at ~6 months and ~12 months[1]

R-MBA Score Mean Change From Baseline: 6 Months



TSHA-102 Mean (N=9, age ≥6 years)	Natural History (N=75, age ≥6 years)
StDev=11.9	StDev=5.97

R-MBA Score Mean Change From Baseline: 12 Months



TSHA-102 Mean (N=4, age ≥6 years)	Natural History (N=196, age ≥6 years)
StDev=4.92	StDev=5.73

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Statistical analyses are based on May 19, 2025, data cutoff: TSHA-102 R-MBA mean 6 months: N=9 (Low dose cohort: N=4, High dose cohort: N=5). TSHA-102 R-MBA mean 12 months: N=4, Low dose cohort.
[1]MBA natural history data converted to R-MBA; mean scores reported were calculated from baseline to 6 and 12 months: Accessed from International Rett Syndrome Foundation (IRSF). ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT.
R-MBA=Revised Motor Behavior Assessment; NHS=Natural history study

TSHA-102 demonstrated early global improvement, with dose-dependent effects deepening over time in CGI-I

Average CGI-I score of 1.0 (*very much improved*) in high dose cohort vs. average CGI-I score of 2.8 in low dose cohort at ≥9 months post-TSHA-102

	3 months	6 months	9 months	12 months	18 months
Low Dose: Average CGI-I Score	**3.0** (N=4)	**2.3** (N=4)	**3.0** (N=2)	**3.3** (N=4)	**2.0** (N=2)
High Dose: Average CGI-I Score	**2.7** (N=6)	**2.0** (N=5)	**1.0** (N=2)		

Time Post TSHA-102:

Based on May 19, 2025, data cutoff. Data presented reflects current data in the Electronic Data Capture System, subject to change.
CGI-I average scores based on latest assessment for each patient. No data available at 12 and 18 months in high dose cohort.
CGI-I=Clinical Global Impression-Improvement

Consistent dose response observed across key measures at 6 months post-TSHA-102, with the separation between dose cohorts increasing over time

Endpoint		Low Dose Cohort	High Dose Cohort	Dose-Dependent Response?
Developmental Milestones	Responder Rate (%)	100% by 12 months	100% by 9 months	✓
	Responder Rate at 6 Months (%)	75%	83%	
R-MBA[1]	Patients with R-MBA Improvement (%) at latest visit	100%	100%	✓
	Mean Score Improvement at 6 Months	-9.8	-12.2	
	Mean Score Improvement at ≥9 Months	-11.5	-18.0	
CGI-I	Patients with CGI-I Improvement (%) at latest visit	75%	100%	✓
	Mean CGI-I Score at 6 Months	2.3	2.0	
	Mean CGI-I Score at ≥9 Months	2.8	1.0	
CGI-S	Patients with CGI-S Improvement (%) at latest visit	25%	33%	✓

Data cut through May 19, 2025. Study is ongoing and data is subject to change.
Low dose data: N=4. High dose data: N=5 at 6 months; N=2 at ≥9 months.
[1]R-MBA mean score change post-TSHA-102 is relative to baseline; lower score=improvement from baseline.

TSHA-102 was generally well tolerated at the low and high dose with no treatment-related SAEs or DLTs

Number of Events Across 12 Pediatric, Adolescent and Adult Patients Dosed in Part A of REVEAL Phase 1/2 Trials

	Low Dose $5.7x10^{14}$ vg (N=4)		High Dose $1x10^{15}$ vg (N=8)		Total (N=12)	
	N	E	N	E	N	E
TEAE Related to TSHA-102:	4	[10]	5	[14]	9	[24]
Serious TEAE Unrelated to TSHA-102:	2	[7]	4	[6]	6	[13]
Serious TEAE Related to TSHA-102:	0	0	0	0	0	0

- All TEAEs related to TSHA-102 were mild-moderate in severity, with the most common being elevated liver enzymes* (N=4, 33%), pyrexia (N=3, 25%), lethargy (N=2, 17%), and elevated levels of NfL in CSF (clinically insignificant) (N=2, 17%)
- Expected transaminase elevations observed
  - Majority experience mild elevations <2x ULN
  - Acute excursions (>5x ULN) less common, clinically asymptomatic and steroid treatment-responsive
- Seizures have generally been well controlled following TSHA-102

*Includes the following: hepatic enzyme increased, hypertransaminasemia, transaminases increased-and liver function test increased

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Data cut through May 20, 2025 (N=12); Patient 11 is 12 weeks post-treatment and Patient 12 is 6 weeks post-treatment. Study is ongoing and data are subject to change.
SAE=Serious adverse event; DLT=Dose-limiting toxicity; TEAE=Treatment-emergent AE; N=Number of participants; E=Number of events; NfL=Neurofilament light chain; CSF=Cerebrospinal fluid; ULN=Upper limit normal

Anticipated TSHA-102 program milestones

Q2 2026	Complete dosing in REVEAL pivotal trial
Q2 2026	Complete dosing in ASPIRE trial
Q2 2026	Report longer-term safety and efficacy data from Part A of the two REVEAL Phase 1/2 trials
Q4 2026	Complete BLA-enabling PPQ campaign for TSHA-102

Well-capitalized through key milestones:

- Cash and cash equivalents of $276.6 million as of March 31, 2026
- Cash runway expected to fund operating expenses and capital requirements **into 2028**

Appendix

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Overview of Clinical Global Impression-Improvement (CGI-I) rating with Rett syndrome-specific anchors[1]

CGI-I: Clinician-rated scale
assessing improvement from baseline

- Designed as a global clinical assessment
- Factors considered to determine change included duration, onset, durability of change and the context of sign/symptom change across the Rett syndrome specific domains of the CGI

Score	CGI-I
1	**Very much improved**
2	**Much improved**
3	**Minimally improved**
4	No change
5	Minimally worse
6	Much worse
7	Very much worse

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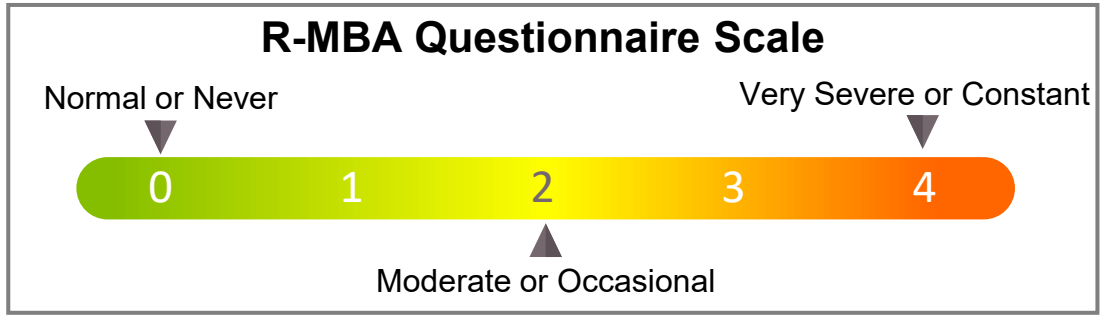
[1]Neul, Jeffrey L et al. "Improving Treatment Trial Outcomes for Rett Syndrome: The Development of Rett-specific Anchors for the Clinical Global Impression Scale." Journal of child neurology vol. 30,13 (2015): 1743-8. doi:10.1177/0883073815579707

Overview of Revised Motor Behavior Assessment (R-MBA)

- Clinician-reported assessment that measures the onset of disease regression, growth, motor and communication skills, and disease behaviors for individuals with Rett syndrome[1]
- **Associated with developmental milestone acquisition and function impacting quality of life**
- Measures the severity or frequency of a diverse set of symptoms to capture phenotypic variability
- Assessed in Rett syndrome natural history study[2]



24-item questionnaire across five domains:

1. Motor Dysfunction
2. Functional Skills
3. Social Skills
4. Aberrant Behavior
5. Rett-specific Behavior (i.e. Respiratory)

[1]Raspa, M. et al. A Psychometric Evaluation of the Motor-Behavioral Assessment Scale for Use as an Outcome Measure in Rett Syndrome. Nov 2020. [2]MBA natural history data converted to R-MBA; Accessed from International Rett Syndrome Foundation (IRSF). ClinicalTrials.gov: NCT02738281: a prospective cohort of individuals with a pathologic mutation in the MECP2 gene, commonly associated with RTT.

Compelling preclinical safety, pharmacology, toxicology & biodistribution data supported clinical advancement of TSHA-102 **in a broad age range of patients**

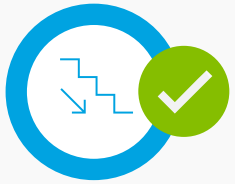
TSHA-102 improved survival, body weight, motor function and respiratory health across all ages evaluated
in KO mouse models of Rett syndrome, and demonstrated a favorable safety profile in KO and WT mice

TSHA-102 was well tolerated up to 2x10^{15} vg
in NHPs and rats

Broad biodistribution to brain and spinal cord
demonstrated in NHPs, rats and mice

WT animals treated with TSHA-102 and vehicle demonstrated similar levels of *MECP2,* supporting the mechanism of miRARE
by minimizing transgene expression in the presence of endogenous *MECP2*

miRARE downregulated *MECP2* transgene and protein expression in response to cellular levels of MeCP2
in human and mouse cell lines



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Source: Company data
KO=Knockout; WT=wild-type; NHP=Non-human primate; vg=Vector genome

Robust preclinical data for TSHA-102 demonstrated **across age ranges**

Species	Animal Model	Age	Study Size	HED (vg/participant)	Route of Administration	Findings:
Mouse	Wild-type and *Mecp2*$^{-/Y}$	Neonates (P2)	n=45	2.9x10^{14}	ICV	○ **Improvement in survival rate, overall neurobehavioral function and growth** in neonatal KO Rett mice ○ No impact on WT treated mice
Mouse	Wild-type and *Mecp2*$^{-/Y}$	P7, P14, P28	n=252	2.9x10^{14} 7.1x10^{14} 1.4 x 10^{15} 2.9x10^{15}	IT	○ **Significant improvement in survival, body weight, motor function and respiratory health** across treatment ages in KO Rett mice ○ No signs of overexpression in WT treated mice
Mouse	Wild-type and *Mecp2*$^{-/Y}$	P28 - P35	n=137	2.9x10^{15}	IT	○ TSHA-102 vector DNA and **transgene distribution demonstrated in the brain and spinal cord** ○ mini*MECP2* RNA detected in brain and spinal cord
Rat	Wild-type	3.4 - 6.1 weeks	n=160	2.5×10^{14} 5.0×10^{14} 2.0×10^{15}	IT	○ **Favorable safety profile** of TSHA-102 ○ Nerve conduction metrics within functional physiological ranges for all groups at all timepoints
Non-human primate	Wild-type	Juvenile (~2 yrs)	n=24	2.5×10^{14} 5.0×10^{14} 2.0×10^{15}	IT	○ TSHA-102 was **well-tolerated with no toxicity observed** ○ **Biodistribution demonstrated in brain and spinal cord, with low *miniMECP2* mRNA expression in the CNS**, indicating miRARE mediated transgene expression in the presence of endogenous *MECP2*
Human and mouse cell lines	2v6.11, SH-SY5Y, and Neuro-2a	NA	NA	NA	Cell transfection and transduction	○ Evidence that **miRARE can control mini*MECP2* transgene and protein expression** in cell culture models ○ miniMeCP2 protein expression **induced by absence of cellular MeCP2**

Source: Company data