A Phase 1/2/3 Study of TSHA-102 Gene Therapy in Females With Rett Syndrome (REVEAL Pivotal Study)

NCT05606614

Status	RECRUITING
Phase	Phase 3
Sponsor	Taysha Gene Therapies, Inc.
Enrollment	15 participants

Key Eligibility Criteria

Inclusion (4)

- Females between the ages of 12 and \<22 in Part A (closed) and females between the ages of 6 and \<22 in Part B (pivotal cohort).
- Participant has a clinical diagnosis of classic/typical Rett syndrome with a documented pathogenic mutation of the methyl-CpG-binding protein 2 (MECP2) gene that results in loss of gene function.
- Participants must be willing to receive blood or blood products for the treatment of an AE if medically needed.
- Participants and parent/caregiver must agree to reside within easy access to the study site prior to the baseline visit and at least 3 months after TSHA-102 treatment

Exclusion (4)

- Participant has another neurodevelopmental disorder independent of the MECP2 loss-of-function mutation, or any other genetic syndrome with a progressive course.
- Participant has a history of brain injury that causes neurological problems or had grossly abnormal psychomotor development in the first 6 months of life.
- Participant has a diagnosis of atypical Rett syndrome or a MECP2 gene mutation that does not cause Rett syndrome.
- Participant requires invasive ventilatory support.

Locations (6 total)

UC San Diego, La Jolla, California, United States
Rush University Medical Center, Chicago, Illinois, United States
Boston Children's Hospital, Boston, Massachusetts, United States
... and 3 more locations