

Rett Syndrome Clinical Trials

There are a variety of trials available and in development. We strongly encourage you to work with your care team to determine which trial is best for your child.

Further details and a complete list of Rett syndrome trials, visit ClinicalTrials.gov.

P = PHASE
 ● = ACTIVE CLINICAL TRIALS
 ● = UPCOMING CLINICAL TRIALS

KETAMINE

- **P2:** Safety, Tolerability, Efficacy
- **Focus:** Improve breathing and motor sensory skills
- **48 Females:** Ages 6-12
- **Status:** Recruiting



SARIZOTAN

- **P2/P3:** Safety, Tolerability and Efficacy
- **Focus:** Improve breathing
- **129 Females:** Ages 4+
- **Status:** Active

TRIHEPTANOIN

- **P2:** Safety and Tolerability
- **Focus:** Increase metabolic profile and motor performance
- **12 Females:** Ages 2+
- **Status:** Recruiting

EPIDIOLEX

- **P3:** Safety and Efficacy
- **Focus:** Reduce symptom severity
- **252 Females:** Ages 2-18
- **Status:** Recruiting

ANAVEX 2-73

- **P2:** Safety
- **Focus:** Improve motor sensory function
- **15 Females:** Ages 18-45
- **Status:** Recruiting/Active

TROFINETIDE

- **P3:** Efficacy
- **Focus:** Improve synaptic function and reduce neuroinflammation
- **180 Females:** Ages 5-20
- **Status:** Estimated Q4 2019

AVEXIS

- Gene therapy
- **Focus:** Correct MeCP2 gene
- **Status:** Pre-clinical development



Rett Syndrome Research Opportunities

NON-DRUG TRIALS: NEURO-HABILITATION TRIALS

TITLE	ELIGIBILITY
Visual Attention to Print in Children with Rett Syndrome	Ages 3-8 years, w/ RTT and their parents
Improving Upper Extremity Motor Skills in Persons with Rett Syndrome Using Virtual Reality Computer Games	The study is to be conducted online from participant's home
Rett Syndrome: Tailoring treatment based on communication needs	Females ages 3-60 years old to participate in 36 in clinic or home sessions that are 60-90 minutes in length
Assessing Oculomotor Control in Rett Syndrome Using Integrated EEG and Eye Tracking Technology	Individuals with RTT aged 2-70 with normal or corrected vision.

NON-DRUG TRIALS: OBSERVATIONAL STUDIES

TITLE	STATUS & ELIGIBILITY
The Natural History Study	Individuals with RTT - all ages
Biobanking of Rett Syndrome and Related Disorders	RECRUITING Females & males, all ages, with a mutation duplication or deletion of MECP2, FOXP1, or CDKL5 OR Meet consensus criteria for RTT (typical or atypical)
Development of a Behavioral Outcome Measure for Rett Syndrome (RettBe)	RECRUITING Females 3 to 18

Clinical trials and studies can and do change. Visit Rettsyndrome.org often for additional information about clinical trials, studies and Natural History Study sites.

