Study of MRM-3379 in Male Participants With Fragile X Syndrome (BLOOM)

Status: RECRUITING

Eligibility Criteria

Sex: MALE
Age: 13 years to 45 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

- Willing and able to provide signed informed consent/assent. Where local regulations permit inclusion of participants deemed unable to provide informed consent, a legally authorized representative must provide informed consent on the participant's behalf, and the participant must provide assent if applicable.
- Male, 13-45 years of age (inclusive)
- Weight ≥30 kg and BMI between 18 and 36 kg/m2 (inclusive) at the screening visit
- Diagnosis of FXS with a molecular genetic ≥200 CGG repetitions .
- Able to perform the PVT and ORRT of the NIH-TCB
- Have a consistent caregiver(s) who is willing and able to be present regularly and reliably with the participant
- Able to swallow tablets or capsules

Exclusion Criteria:

History of or current medical condition other than FXS and related issues that would place the participant at higher risk from study participation

Conditions & Interventions

Interventions:
DRUG: Low dose of MRM-3379, DRUG: Middle Dose of MRM-3379, DRUG: High dose of MRM-3379, DRUG: Placebo, DRUG: Low dose of MRM-3379 Open-Label
Conditions:
Fragile X Syndrome
Keywords:
Fragile X Syndrome, FXS, FMR, Phosphodiesterase 4, PDE4

More Information

Description:
This study is a multicenter, double-blind, randomized, placebo-controlled study to assess the safety and tolerability of 3 doses of MRM-3379 in male participants with Fragile X Syndrome ages 16 to 45 (inclusive). In addition, a parallel cohort of participants ages 13 to \<16 will receive open-label MRM-3379. All participants will participate for 12 weeks of treatment. The study is also intended as a proof-of-concept investigation to evaluate whether MRM-3379 can improve FXS symptoms

Contact(s): Hannah Sachs - Hannah.Sachs@cchmc.org
Principal Investigator:
Principal Investigator ID:
Phase: PHASE2
IRB Number:
System ID: NCT07209462