A Phase 1/2 Study of the Safety and Efficacy of MVX-220 in Angelman Syndrome

NCT07181837

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	MavriX Bio, LLC
Enrollment	12 participants

Key Eligibility Criteria

Inclusion (8)

- The participant's parent/legal guardian must provide written informed consent.
- Symptoms consistent with AS and documented genetic confirmation of one of the following genotypes resulting in a diagnosis of AS:
- Full maternal UBE3A gene deletion causing AS in the region of 15q11.2-q13
- Uniparental disomy
- Imprinting center defect

... and 3 more (see full listing online)

Exclusion (21)

- Clinically significant medical finding other than AS, that, in the judgment of the Investigator would make the participant unsuitable for participation.
- Laboratory abnormalities including but not limited to:
- Alanine aminotransferase (ALT) or aspartate aminotransferase (AST) \> upper limit of normal (ULN)
- Total and/or fractionated bilirubin (direct and/or indirect) \> ULN
- Gamma-glutamyl transferase (GGT) \> ULN

... and 16 more (see full listing online)

Locations (3 total)

Cedars-Sinai Medical Center, Los Angeles, California, United States
Rush University Medical Center, Chicago, Illinois, United States
Boston Children's Hospital, Boston, Massachusetts, United States