A Safety and Efficacy Study of GTX-102 in Subjects With Deletion- or Nondeletion-type Angelman Syndrome (AS)

NCT07157254

Status	RECRUITING
Phase	Phase 2
Sponsor	Ultragenyx Pharmaceutical Inc
Enrollment	60 participants

Key Eligibility Criteria

Inclusion (10)

- Signed informed consent from parent(s) or legal guardian(s)
- Males and females of the following ages and genotypes at time of informed consent:
- Subprotocol A: e 1 to \< 4 years of age with a genetically confirmed diagnosis of deletion-type Angelman syndrome
- Subprotocol B: e 4 to \< 18 years of age with a genetically confirmed diagnosis of UPD/ICD Angelman syndrome
- Subprotocol C: e 18 to \< 65 years of age with a genetically confirmed diagnosis of Angelman syndrome, any genotype

... and 5 more (see full listing online)

Exclusion (8)

- Any change in medications or diet/supplements intended to treat symptoms of Angelman Syndrome (eg, sleeping aids, antiseizure medications, supplements, dietary change including ketogenic or low-glycemic index diet, other) within the month prior to the Screening Visit (excluding weight-based adjustments)
- Any condition that creates an increased risk of unsuccessful lumbar puncture
- Current or expected concomitant use of drugs that increase the risk of bleeding (eg, heparin, low molecular weight heparin, platelet inhibitors)
- Known hypersensitivity to GTX-102 or its excipients or required premedication that, in the judgment of the Investigator, places the subject at increased risk for adverse effects
- Presence or history of any condition, lab abnormality, or infection that, in the judgment of the Investigator, would interfere with study participation, pose undue safety risk, or would confound interpretation of results

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

Locations (22 total)

Clinical Trial Site, Los Angeles, California, United States
Rush University Medical Center, Chicago, Illinois, United States
Clinical Trial Site, Baltimore, Maryland, United States
... and 19 more locations

DISCLAIMER: This document is for informational purposes only and does not constitute medical advice. Always consult your healthcare provider before enrolling in any clinical trial. Information may not be up to date — verify details at ClinicalTrials.gov. Generated by ClinicalTrialsFinder.org.