

REVEAL: A Phase 3 Study of ION582 in Angelman Syndrome

NCT06914609

Status	RECRUITING
Phase	Phase 3
Sponsor	Ionis Pharmaceuticals, Inc.
Enrollment	158 participants

Key Eligibility Criteria

Inclusion (6)

- The participants caregiver(s)/ legally authorized representative must have given written informed consent and any authorizations required by local law and be able to comply with all study requirements.
- Medically stable and can undergo sedation and/or general anesthesia without intubation.
- Male or female between 2 and lesser than or equal to (d)50 years of age, depending on specific cohort, at the time of the in-clinic Screening visit.
- Participant has a clinical diagnosis of Angelman syndrome (AS) with molecular confirmation of either Ubiquitin-protein ligase E3A (UBE3A) deletion or UBE3A mutation.
- Currently receiving stable doses of concomitant medications typically prescribed for AS, such as anti-epileptic medication, behavioral management medications, sleep medications, gabapentin, cannabidiol, and special diets, supplements, or nutritional support for at least 8 weeks prior to the Baseline visit.

... and 1 more (see full listing online)

Exclusion (6)

- Must not have any clinically significant abnormalities in medical history (e.g., major surgery within 3 months of screening), or on physical examination for which treatment with an antisense oligonucleotide (ASO) would be contraindicated or which, in the opinion of the Principal Investigator (PI), could confound the results of this study.
- Known brain or spinal disease that would interfere with the lumbar puncture (LP) procedure, cerebrospinal fluid (CSF) circulation, or presence of other factors would affect the safety of the LP procedure.
- Must not have any other conditions, which, in the opinion of the Investigator, would make the participant unsuitable for inclusion or could interfere with the participant participating in or completing the study.
- Must not have any laboratory abnormalities or any other clinically significant abnormalities that would, as assessed by the Investigator, at screening or Baseline, render a participant unsuitable for inclusion.
- Previous treatment with an oligonucleotide (including small interfering ribonucleic acid (RNA) \[siRNA\], ASOs) gene therapy or gene editing. This exclusion criterion does not apply to approved nucleic acid-based vaccines, including mRNA vaccines, which are allowed.

... and 1 more (see full listing online)

Locations (36 total)

Cedars-Sinai Medical Center, Los Angeles, California, United States
Rady Children's Hospital, San Diego, California, United States
Colorado Children's Hospital Research Institute, Aurora, Colorado, United States
... and 33 more locations

<https://clinicaltrials.gov/study/NCT06914609>

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