

CHAMPION: A Phase 3 Study to Evaluate the Efficacy and Safety of Obudanersen (ION582) in Children and Adults With Angelman Syndrome (AS) Due to Paternal Uniparental Disomy or Imprinting Defects¹

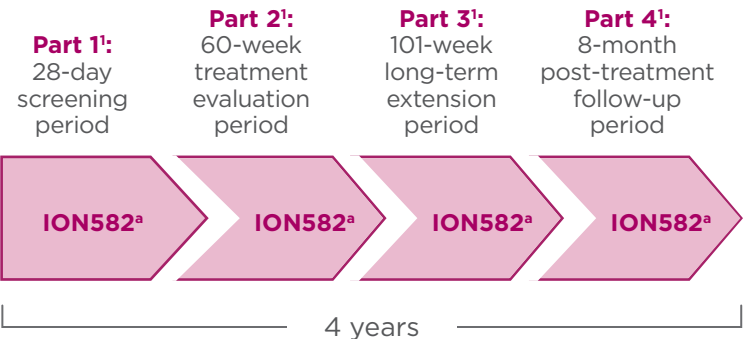


Study objective:

To evaluate the efficacy of obudanersen (ION582) in participants with AS due to paternal uniparental disomy (UPD) or imprinting defects (IDs), as measured through expressive communication. The study will also evaluate the safety of obudanersen and additional clinical outcomes.¹



N=30
participants
(estimated)



This is a multicenter, open-label, single-arm Phase 3 study in participants with AS due to paternal UPD or IDs. The study consists of a screening period of up to 28 days, an approximately 60-week treatment evaluation period, a 101-week long-term extension (LTE) period, and an 8-month post-treatment follow-up period. Two age-based cohorts will be enrolled: Cohort 1 (participants aged 2 to <18 years) and Cohort 2 (participants aged 18 to ≤50 years). More participants will be enrolled in Cohort 1 than in Cohort 2.¹

Select inclusion/exclusion criteria^{1,b}:

- Male or female aged 2 to ≤50 years¹
- Clinical diagnosis of AS with molecular confirmation of either paternal UPD of 15q11.2-q13 or ID of the maternal 15q11.2-q13 region¹
- Medically stable and able to undergo sedation and/or general anesthesia without intubation¹
- Participants with molecular confirmation of a *UBE3A* deletion or *UBE3A* mutation are excluded¹
- Participants with clinically significant abnormalities rendering them unsuitable for participation are excluded^{1,c}

For more study information, scan here:



All information is accurate as of 09/2026. For the most up-to-date information, please scan QR code.

Key Clinical Endpoints ^{1,d}	
Primary Endpoint	Change from baseline in performance on the Expressive Communication Domain Raw Score without caregiver input of the Bayley Scales of Infant and Toddler Development-4 (Bayley-4) in Cohort 1 (Baseline to Week 52)
Secondary Endpoints	• Change from baseline in Bayley-4 Cognition Scale Raw Score without caregiver input (Baseline to Week 52) • Change from baseline in Aberrant Behavior Checklist, Second Edition Community Version (ABC-2-C) Hyperactivity score (Baseline to Week 52) • Change from baseline in ABC-2-C Irritability score (Baseline to Week 52) • Change from baseline in Vineland Adaptive Behavior Scale-3 (Vineland-3) Receptive Communication Domain Raw Score (Baseline to Week 52) • Change from baseline in Vineland-3 Daily Living Skills, Personal Domain Raw Score (Baseline to Week 52) • Change from baseline in Observer-Reported Communication Ability (ORCA) Overall Emerging and Mastery T Score (Baseline to Week 52)



Obudanersen has not been evaluated for safety and efficacy by any regulatory authorities and is not indicated for the treatment of any disease.

^aAdministered by lumbar intrathecal bolus injection.¹ ^bThis is not an exhaustive list of eligibility criteria. Additional protocol-defined inclusion and exclusion criteria apply. For complete eligibility information, please scan the QR code above.¹ ^cThese include, but are not limited to, known brain or spinal disease that would interfere with the lumbar puncture procedure or cerebrospinal fluid circulation, clinically significant medical history findings, laboratory abnormalities, prior oligonucleotide therapy, gene therapy, or gene-editing treatment.¹ ^dThis is not an exhaustive list.¹
UBE3A; ubiquitin protein ligase E3A gene.

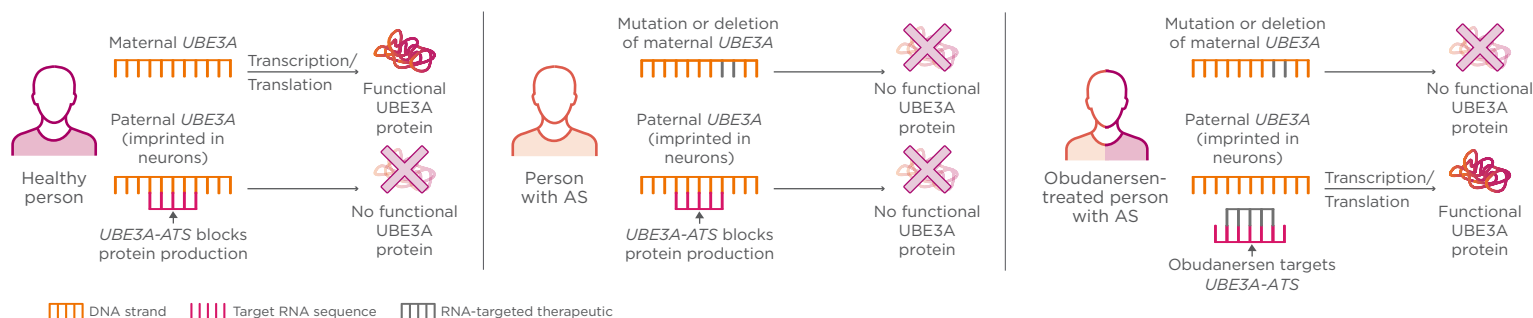
1. ClinicalTrials.gov identifier: NCT07782827. Accessed August 25, 2026. <https://clinicaltrials.gov/study/NCT07782827>

Obudanersen Is an Investigational RNA-Targeted Therapeutic (RTT) Designed to Increase Neuronal Expression of *UBE3A*¹



- In Angelman syndrome, *UBE3A* expression is lost on the maternal gene. *UBE3A* expression is healthy on the paternal strand but silenced by *UBE3A-ATS*²
- The loss of *UBE3A* expression is the cause of many symptoms associated with Angelman syndrome²

Proposed Obudanersen-Mediated Upregulation of *UBE3A*¹⁻³



RTTs downregulate *UBE3A-ATS*, unsilencing paternal *UBE3A* expression and restoring brain-wide *UBE3A* protein levels in mouse models²⁻⁴

Administration of a single dose of *UBE3A-ATS*-targeting antisense RTT in mouse models reduced *UBE3A-ATS* levels in the CNS for 16 weeks.⁴

Both *UBE3A* mRNA and *UBE3A* protein levels were significantly higher in RTT-treated mice than in control mice from 2 to 16 weeks after treatment.⁴



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For more information or questions about participating sites, please contact us at Champion@clinicaltrialmedia.com or 844-373-1141.⁵

AS, Angelman syndrome; CNS, central nervous system; mRNA, messenger RNA; *UBE3A*, ubiquitin protein ligase E3A protein; *UBE3A*, ubiquitin protein ligase E3A gene; *UBE3A-ATS*, ubiquitin protein ligase E3A-antisense transcript gene.

1. Ionis Pharmaceuticals. Pipeline. Accessed August 25, 2026. <https://www.ionis.com/science-and-innovation/pipeline>. 2. O'Geen H, et al. *Mol Ther*. 2023;31(4):1088-1105. 3. Milazzo C, et al. *JCI Insight*. 2021;6(15):e145991. 4. Meng L, et al. *Nature*. 2015;518(7539):409-412. 5. ClinicalTrials.gov identifier: NCT07782827. Accessed August 25, 2026. <https://clinicaltrials.gov/study/NCT07782827>