



FOR 15Q 11.2-13.1 MULTIPLICATIONS

# Research Symposium 2025

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**JULY 21 – 23, 2025**

**SHERATON INDIANAPOLIS HOTEL AT KEYSTONE CROSSING  
8787 KEYSTONE CROSSING, INDIANAPOLIS, INDIANA, USA, 46240**

# MONDAY

TIME	TITLE	PRESENTER
9:00 AM - 9:20 AM	WELCOME AND OPENING REMARKS	
SESSION 1		
9:20 AM - 9:50 AM	Targeting Transferrin Receptor to Deliver Antisense Oligonucleotides Across the Blood-Brain Barrier	Miyamoto
9:55 AM - 10:25 AM	A Novel Mouse Model for Duplication 15q11.2-13 Illustrates Construct and Face Validity: Critical for Biomarker & Therapeutics	Silverman
10:30 AM - 10:45 AM	An Update on Biomarker Development for Angelman Syndrome	Mathews
10:50 AM - 11:05 AM	Hematopoietic Stem Cell Gene Therapy as a Novel Therapeutic Approach for Angelman Syndrome	Anderson
SESSION 2		
11:35 AM - 12:05 PM	Comparative In Vitro and In Vivo Analysis of ASOs Targeting UBE3A-ATS	Chamberlain
12:10 PM - 12:25 PM	A Humanized Ube3a-ATS Mouse Model to Evaluate and Screen Therapeutics for Angelman Syndrome	James
12:25 PM - 1:45 PM	LUNCH BREAK	
SESSION 3		
1:45 PM - 2:15 PM	Preclinical Development of a Gene Therapy for Angelman Syndrome	Judson
2:20 PM - 2:35 PM	UBE3A Isoform-Selective and Non-Selective Contributions to Angelman Syndrome Phenotypes	Krzeski
2:40 PM - 2:55 PM	Progress Toward a Small Molecule Therapeutic for Angelman Syndrome	Welton-Arndt and Nourie
SESSION 4		
3:00 PM - 3:30 PM	Non-Viral Delivery of Genome Medicine to the Central Nervous System	Zhou
3:35 PM - 4:05 PM	A New Home for Rugonersen: Advancing a Potential Treatment for Angelman Syndrome	Tjeertes
4:10 PM - 4:40 PM	GI Problems in a Mouse Model of Angelman Syndrome: From Bench to Bedside	Woo
4:45 PM - 5:00 PM	Increased Extra-Axial Cerebrospinal Fluid Volume in Children with Angelman Syndrome: Links to Sleep Problems and Seizures	Shen

# TUESDAY

TIME	TITLE	PRESENTER
8:30 AM - 8:45 AM	DAY 2 OPENING REMARKS	
SESSION 5		
8:45 AM - 9:15 AM	Characterization of Novel Mouse Models for Dup15q	Elgersma
9:20 AM - 9:50 AM	Biochemical and Biophysical Approaches to Study UBE3A Structure/Function	Scheffner
9:55 AM - 10:25 AM	Clinical and Research Applications of Long-Read Sequencing for Suspected Imprinting Conditions	Miller
SESSION 6		
10:55 AM - 11:25 AM	Development of an ASO Targeting UBE3A for Dup15q Syndrome Using a Proprietary AI-Enabled CNS Drug Discovery Platform	Fink
11:30 AM - 12:00 PM	Biosensor to Measure Endogenous UBE3A Activity	Zylka
12:00 PM - 1:15 PM	LUNCH BREAK	
SESSION 7		
1:15 PM - 1:45 PM	Phenotypic Characterization of UBE3A Gain-of-Function Animals	Yi
1:50 PM - 2:20 PM	A Novel Inflammatory Signature in dup15q	Dias
SESSION 8		
2:50 PM - 3:20 PM	Compound Screening for New Dup15q Anti-Seizure Drugs Using Drosophila Melanogaster	Reiter
3:25 PM - 3:40 PM	Identification of UBE3A Substrates Through an In Vivo Ubiquitin-Activated Interaction Trap (UBAIT) Approach in Drosophila	Geier
SESSION 9		
4:10 PM - 4:25 PM	Periodic and Aperiodic Components of Elevated Delta Power in Angelman Syndrome	Montgomery
4:30 PM - 4:45PM	Machine Learning Based Classification of EEG Activity in Angelman Syndrome	Hodges
4:50 PM - 5:05 PM	Outcome Measures in Angelman Syndrome	Hagenaar
5:10 PM - 5:40 PM	Innovation & Investment in Biotech: Implications for New Therapies for Neurodevelopmental Disorders	Ehlers
6:15 PM - 8:15 PM	RECEPTION & POSTER PRESENTATION	

# WEDNESDAY

TIME	TITLE	PRESENTER
8:00 AM - 8:30 AM	CLOSING BREAKFAST WITH ASF & DUP15Q ALLIANCE	
8:30 AM - 8:40 AM	DAY 3 OPENING REMARKS	
SESSION 10		
8:40 AM - 9:10 AM	Goodbye "Deletion vs. Non-Deletion," Hello to Genotype-Specific Analyses in Angelman Syndrome	Garbarini & Armstrong
9:15 AM - 9:45 AM	Advancing Angelman Syndrome Research: Real-World Data, Regulatory Readiness, and Early Diagnosis Initiatives	Wheeler
SESSION 11		
9:50 AM - 10:30 AM	Voices of Families	Dup15q & Angelman Syndrome Families
SESSION 12		
11:00 AM - 11:30 AM	Characterization of Nonepileptic Myoclonus in Angelman Syndrome, the Next Steps.	Carson
11:35 AM - 11:50 AM	Eye Tracking for Detection of Cerebral/Cortical Visual Impairment (CVI) in Children with 15q Disorders – Vision In SyndrOmic Neurodevelopmental Disorders-15q (VISION-15q) Study	Chang
11:55 AM - 12:10 PM	Speech and Language in Children with Dup15Q Syndrome	Navis
12:10 PM - 12:15 PM	CLOSING REMARKS	
12:30 PM - 2:00 PM	LLN CLINICIAN MEETING (WITH LUNCH)	

