



2025 global science summit

fast  **Dream
Big.**



Focus on Angelman Syndrome Translational Research

Breakfast & Basics: Understanding Research – Empowering Decisions		
8:30 AM	What Does “In-Vitro” Mean and Why Do We Need These Tools?	Michael Boland, PhD <i>University of Pennsylvania Perelman School of Medicine</i>
9:00 AM	What Does “In-Vivo” Mean and Why Do Models Matter in AS?	Rodney Samaco, PhD <i>Rare Collective Strategies LLC</i>
9:30 AM	What is UBE3A and How Does it Function?	Seth S. Margolis, PhD <i>Johns Hopkins University School of Medicine</i>
10:00 AM	Introduction to Gene Therapy	Emma James, DPhil <i>Encoded Therapeutics</i>
10:30 AM	Questions/Discussion	Allyson Berent, DVM, DACVIM <i>FAST and AS²Bio</i>
10:45 AM	Break	
11:00 AM	Roadmap to a Cure 4.0: From Bench to Bedside	Allyson Berent, DVM, DACVIM <i>FAST and AS²Bio</i>
11:55 AM	Recap of Closed-Door Scientific Symposium: Angelman Syndrome Biomarker and Outcome Measure Consortium (A-BOM) and Translational Research Symposium (TRS)	Allyson Berent, DVM, DACVIM <i>FAST and AS²Bio</i> <i>Jennifer Panagoulas, RAC FAST and AS²Bio</i>
12:15 PM	Biomarkers: EEG, Blood and Fluids	Jennifer Panagoulas, RAC <i>FAST and AS²Bio</i>
12:30 PM	Lunch	
1:10 PM	Lunch and Legislation	Ryan Fischer <i>FAST</i>
Translational Research Presentations: Progress in Research & Discovery!		
1:40 PM	Patient Cell Lines and How They are Used for the Development of Therapies for Angelman Syndrome	Yong-Hui Jiang, MD, PhD <i>Yale School of Medicine</i>
1:55 PM	The New Large Deletion Mouse Model: A Valuable Tool for Angelman Syndrome Research	Xiaona Lu, MD, PhD <i>Yale School of Medicine</i>
2:10 PM	Using CRISPR Activation to Address the Genes Outside of UBE3A for Individuals with Deletions	Nadav Ahituv, PhD <i>University of California, San Francisco</i>

2:25 PM	AI-Powered Drug Discovery Identifies Repurposed Candidates for the Treatment of Angelman Syndrome	Wayne Chadwick, PhD <i>Healx</i>
2:35 PM	Adaptive Functioning in Individuals with Angelman Syndrome	Sarah Nelson Potter, PhD, CCC-SLP <i>RTI International</i>
2:45 PM	Best Practices for Caregivers to Support your Child During the Bayley and Vineland Assessments in Angelman Syndrome Clinical Trials	Anne C. Wheeler, PhD <i>RTI International</i>
2:55 PM	The MDRI Framework: Capturing Meaningful Change Across Symptom Domains that Matter to Patients	Kimberly Goodspeed, MD <i>Ultragenyx</i>
3:05 PM	ORCA and What Meaningful Change Looks Like from a Parent’s Perspective	Abigail Rader <i>Duke University</i>
3:15 PM	Screening for Cerebral Visual Impairment (CVI) in AS	Emma Bauschard <i>Boston Children’s Hospital</i>
3:25 PM	FAST Without Borders: Global Affiliate Highlights	Ellen Koekoekx <i>FAST</i>
3:40 PM	Break	
Oral Poster Session Presentations		
3:50 PM	From the Blood to the Brain: Stem Cell Gene Therapy	Colin Juett <i>UCLA</i>
3:55 PM	Cracking the Code: Understanding <i>UBE3A</i> Mutations	Niki Armstrong, MS, CGC <i>FAST</i>
4:00 PM	Understanding GI Challenges in Angelman Syndrome: What Families Can Learn from the Natural History Study	Adriana Gomes, MD <i>Rady Children’s Hospital/University of California, San Diego</i>
4:05 PM	Project WellCAST: Personalizing Mental Wellness Support for Angelman Caregivers	Bridgette Kelleher, PhD <i>Purdue University</i>
4:10 PM	Top Caregiver-Reported Concerns in Children and Adults with Angelman Syndrome	Madelaine Gramling <i>Boston Children’s Hospital</i>
4:15 PM	Development of the Measure of Observable Outcomes of Distress in Angelman Syndrome (MOOD-AS)	Anne C. Wheeler, PhD <i>RTI International</i>
4:20 PM	New Tools for Assessing Therapeutic Activity and Efficacy	Albert Keung, PhD <i>North Carolina State University</i>
4:25 PM	Measuring UBE3A in Cerebrospinal Fluid	Morty Razavi, PhD <i>SISCAPA Assay Technologies</i>
4:30 PM	Update on Angelman CSF UBE3A Biomarker	Joel Mathews, PhD <i>Ionis Pharmaceuticals</i>
4:45 - 6:00 PM	Family Poster Session Happy Hour	

Focus on Clinical Trials

Time	Talk Title	Pillar	Speaker
8:30 AM	Keynote: Gene Therapy Using Hematopoietic Stem Cells	1	Donald B. Kohn, MD <i>University of California, Los Angeles</i>
9:20 AM	MVX-220: A Gene Replacement Therapy for Angelman Syndrome	1	James M. Wilson, MD, PhD <i>GEMMABio</i>
			Elizabeth Berry-Kravis, MD, PhD <i>Rush University Medical Center</i>
9:45 AM	A Novel Path to the Brain: Crossing the Blood Brain Barrier for Gene Therapies	1, 2	Andrew Steinsapir, MBA <i>Apertura Gene Therapy and Deerfield Management</i>
9:55 AM	Novel Gene Editing Approach for the Potential Treatment of Angelman Syndrome	2	Raphael Bendriem, PhD <i>Arbor Biotechnologies</i>
10:05 AM	CRG-AS-001: A Non-Viral Delivered CRISPR Gene Editing Approach for AS: From Bench to Bedside	2	Yong-Hui Jiang, MD, PhD <i>Yale School of Medicine</i>
			Jiangbing Zhou, MD, PhD <i>Yale School of Medicine</i>
			Allyson Berent, DVM, DACVIM <i>FAST and AS²Bio</i>
10:25 AM	What Parents Want to Know About Ongoing and Upcoming Clinical Trials: Dosing, Schedules, Etiquette and More	5	Allyson Berent, DVM, DACVIM <i>FAST and AS²Bio</i>
10:45 AM	Break		

Time	Talk Title	Pillar	Speaker
11:00 AM	The BEACON Phase 3 Study of Rugonersen – Lighting the Way for Angelman Syndrome	2	Brenda Vincenzi, MD <i>Oak Hill Bio</i>
11:20 AM	GTX-102 -- What We've Learned Together	2	Kimberly Goodspeed, MD <i>Ultragenyx</i>
11:55 AM	Update on the Ionis Development Program for Angelman Syndrome	2	Candice Junge, PhD <i>Ionis Pharmaceuticals</i>
12:30 PM	An Update on the Phase 1/2 Trial Using Alogabat	4	Shady Sedhom, PhD <i>F. Hoffmann-La Roche Ltd</i>
12:40 PM	Wrap Up and Q & A		Allyson Berent, DVM, DACVIM <i>FAST and AS²Bio</i>