

Program Schedule

Friday, November 6

8:00 a.m.	Breakfast	
8:30 a.m.	Roadmap to a Cure: Progress and Possibilities	Allyson Berent, DVM, DACVIM <i>FAST and AS²Bio</i>
9:30 a.m.	UBE3A and Beyond: What You Need to Know About Therapeutic Options and Your Child's Genotype	Niki Armstrong, MS, CGC <i>FAST</i>
10:00 a.m.	Function of UBE3A Isoforms: Isoform 1, 2: What Does This Mean	Seth S. Margolis, PhD <i>Johns Hopkins University School of Medicine</i>
10:15 a.m.	Break	
		Cesar Ochoa-Lubinoff, MD, MPH <i>Cedars-Sinai</i>
10:35 a.m.	Standards of Care Today in 2026: Where Are We Today and Where Are We Going Tomorrow	Lynne Bird, MD <i>Rady Children's Health/University of California, San Diego</i> Jean Baptiste Le Pichon, MD, PhD <i>Children's Mercy</i>
11:20 a.m.	The Drug Approval Process: What, How and Why Does It Take So Long?	Jennifer Panagoulas, RAC <i>FAST and AS²Bio</i>
11:45 a.m.	Post-Trial Expectations: Preparing for All Scenarios	Ryan Fischer <i>FAST</i>
12:05 p.m.	GTX-102: What We Are Doing to Understand the Results and What Comes Next	Allyson Berent, DVM, DACVIM Elizabeth Berry-Kravis, MD, PhD <i>Rush University Medical Center</i>
12:25 p.m.	Lunch	
1:05 p.m.	20th Anniversary of the Angelman Syndrome Natural History Study	Anjali Sadhwani, PhD <i>Boston Children's Hospital</i> Wen-Hann Tan, BMBS <i>Boston Children's Hospital</i>
1:15 p.m.	Hope in Action: Update on Federal and State Advocacy Efforts	Ryan Fischer <i>FAST</i>
1:35 p.m.	One Mission, Global Impact: Bringing Progress to Angelman Families Around the World	Ellen Koekoekx <i>FAST</i>
1:50 p.m.	Recap of Translational Research Symposium and A-BOM	Allyson Berent, DVM, DACVIM Jennifer Panagoulas, RAC
2:05 p.m.	What Matters to Families: What Do These Scores Really Mean? ORCA, ABC-C, Bayley, ASA... Oh My!	Allyson Berent, DVM, DACVIM Jennifer Panagoulas, RAC Parent Panel
2:45 p.m.	Expressive Communication in Angelman Syndrome: Analysis of the Data from the Angelman Syndrome Natural History Study	Caroline Woeber, MA, CCC-SLP <i>Children's Hospital Colorado</i>
2:55 p.m.	Question and Answer from Pharma to Families: Let US Help YOU	Allyson Berent, DVM, DACVIM Ryan Fischer

Pre-Clinical Therapeutic Programs

3:15 p.m.	What Genes Matter Outside of <i>UBE3A</i> ? CRISPR Activation and the Deletion Genotype	Nadav Ahituv, PhD <i>University of California, San Francisco</i> Yong-Hui Jiang, MD, PhD <i>Yale School of Medicine</i>
3:40 p.m.	Zinc Fingers: Are We There Yet?	David J. Segal, PhD <i>University of California, Davis</i>
3:50 p.m.	Break	
Scientific Poster Session for Families: 5-Minute Fire Round		
4:10 p.m.	Contribution of GABA-A Receptor Subunit Genes to Angelman Syndrome Pathophysiology	Eric Levine, PhD <i>University of Connecticut School of Medicine</i>
4:15 p.m.	How Neurons and Brain Immune Cells Talk in Angelman Syndrome: A New Window into <i>UBE3A</i> , Inflammation, and Synapses	Yu-Wen Alvin Huang, MD, PhD <i>Brown University</i>
4:20 p.m.	Multiple Cell Specific Proteomics Identifies <i>UBE3A</i> Substrates Relevant to Angelman Syndrome	Benjamin Geier <i>Tulane University</i>
4:25 p.m.	Antibody-Mediated Blood-Brain Barrier Modulation Enables Systemic Delivery of Antisense Oligonucleotides Treatment for Angelman Syndrome Mouse Model	Xiaona Lu, PhD <i>Yale School of Medicine</i>
4:30 p.m.	CRISPR-Cas13d Effectively Unsilences Paternal <i>UBE3A</i> in Angelman Human Neurons and Young Rhesus Macaques	Adele Guglielmi, PhD <i>University of California, Davis</i>
4:35 p.m.	Design and Screening of Divalent Silencing RNA's to Silence Paternal <i>UBE3A</i> -ATS and Reactivate Paternal <i>UBE3A</i> in Human and Mouse Models of Angelman Syndrome	Mark Deehan, PhD <i>Massachusetts General Hospital</i>
4:40 p.m.	ZIP- <i>UBE3A</i> : The Backstage Pass to the Brain	Barbara Bailus, PhD <i>Keck Graduate Institute</i>
4:45 p.m.	ABO-205: A Novel Gene-Editing Therapeutic Program as a Potential Treatment for Angelman Syndrome	Raphael Bendriem, PhD <i>Arbor Biotechnologies</i>
4:50 p.m.	Development of a Modular Recombinant <i>UBE3A</i> Platform for Biochemical Characterization and Non-Viral Cellular Delivery Studies	Celina Maria Enriz <i>Universidad de Buenos Aires</i>
4:55 p.m.	Investigating the Neuronal Origin of <i>UBE3A</i> in Cerebrospinal Fluid	Morty Razavi, PhD <i>SISCAPA Assay Technologies</i>
5:00 p.m.	Establishing a Gene Therapy Development Framework for TfR1 CapX, an AAV Platform for Neurological and Neurometabolic Disorders	Andrew Steinsapir, MBA <i>Apertura Gene Therapy and Deerfield Management</i>
5:05 p.m.	Poster Reception	

Saturday, November 7

8:00 a.m.	Breakfast	
8:30 a.m.	Parallel Paths: Building Gene Therapy From the Ground Up	James Wilson, MD, PhD <i>Gemma Bio</i>
9:00 a.m.	Ubecel: HSC Gene Therapy Program Update	Jennifer Panagoulas, RAC <i>AS²Bio and TransformaTx Bio</i>
9:20 a.m.	MXV-220: A Gene Replacement Therapy for Angelman Syndrome	Serena Hung, MD <i>MavriX Bio</i>
9:45 a.m.	CRG-AS-001: A Non-Viral Delivered CRISPR Gene Editing Approach for AS... Within Reach	Yong-Hui Jiang, MD, PhD <i>CourageAS Bio</i> Elizabeth Berry-Kravis, MD, PhD <i>Rush University Medical Center</i> Allyson Berent, DVM, DACVIM <i>AS²Bio and CourageAS</i>
10:10 a.m.	Developing an AAV-miRNA Approach to Upregulate UBE3A for the Treatment of Angelman Syndrome	Sirika Pillay, PhD <i>Encoded Therapeutics</i>
10:35 a.m.	Break	
10:55 a.m.	BEACON: A Phase 3 Study of Rugonersen - An Update to the Angelman Syndrome Community	Brenda Vincenzi, MD <i>Oak Hill Bio</i>
11:20 a.m.	Update on the Ionis Development Program for Angelman Syndrome	Rob Komorowski, PhD <i>Ionis Pharmaceuticals</i>
11:45 a.m.	Ultragenyx Update	Kimberly Goodspeed, MD <i>Ultragenyx</i>
12:20 p.m.	Community Questions	Allyson Berent, DVM, DACVIM <i>FAST and AS²Bio</i>
