

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, 3: What Does This Mean	Seth S. Margolis, PhD <i>Johns Hopkins University School of Medicine</i>
10:15 a.m.	Break	
10:35 a.m.	Standards of Care Today in 2026: Where Are We Today and Where Are We Going Tomorrow	Cesar Ochoa-Lubinoff, MD, MPH <i>Cedars-Sinai</i> 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
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: The 10th Anniversary of Shared Learnings	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 Parent Panel

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 UBE3A, Inflammation, and Synapses	Yu-Wen Alvin Huang, MD, PhD <i>Brown University</i>
4:20 p.m.	Multiple Cell Specific Proteomics Identifies UBE3A 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 Oligonucleotide 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-ATS</i> 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.	Backstage Pass to the Brain: The Mouse Tour!	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 UBE3A 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 UBE3A 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 for Questions and Engagement	

Following the conclusion of Friday’s Global Science Summit, we invite you to join us for a reception featuring the following posters:

- 1. Diagnostic Trends and Challenges in UPD and ICD Angelman Syndrome: Insights from GASR**
Niki Armstrong, MS, CGC (*FAST*)
- 2. Designing a Minimally Invasive Enzyme Replacement Therapy for Angelman Syndrome**
Barbara Bailus, PhD (*Keck Graduate Institute*)
- 3. Backstage Pass to the Brain: The Mouse Tour!**
Barbara Bailus, PhD (*Keck Graduate Institute*)
- 4. ABO-205: A Novel Gene-Editing Therapeutic Program as a Potential Treatment for Angelman Syndrome**
Raphael Bendriem, PhD (*Arbor Biotechnologies*)
- 5. Early Evidence for Conditioning-Free Hematopoietic to Microglia-Like Cell Transitioning In Vivo**
Rachel Cunningham, PhD (*University of Massachusetts Chan Medical School*)
- 6. A Novel, App-Aided, Non-Invasive, Whole-Gut Transit Measure in People with Autism and Neurodevelopmental Disorders**
Julia Eve Dallman, PhD (*University of Miami*)
- 7. Design and Screening of Divalent Silencing RNA’s to Silence Paternal *UBE3A-ATS* and Reactivate Paternal *UBE3A* in Human and Mouse Models of Angelman Syndrome**
Mark Deehan, PhD (*Massachusetts General Hospital*)
- 8. Developing a Long-Lasting Stem Cell Gene Therapy for Angelman Syndrome**
Laurence Fairchild, MEng (*University of California, Los Angeles*)
- 9. Engineering HSC-Derived Microglia-Like Cells for Sustained *UBE3A* Delivery for Angelman Syndrome**
Laurence Fairchild, MEng (*University of California, Los Angeles*)
- 10. Clinical and Nutritional Phenotypes Across Age and Sex in Brazilian Individuals with Angelman Syndrome: Findings from the First National Survey**
Ana Luisa Kremer Faller, PhD (*Nutrition Institute Josue de Castro at Federal University of Rio de Janeiro*)
- 11. Angelman Syndrome Biomarker Discovery Using *Drosophila* Hemolymph Metabolomics**
Benjamin Geier (*Tulane University*)
- 12. Multiple Cell Specific Proteomics Identifies *UBE3A* Substrates Relevant to Angelman Syndrome**
Benjamin Geier (*Tulane University*)
- 13. CRISPR-Cas13d Effectively Unsilences Paternal *UBE3A* in Angelman Human Neurons and Young Resus Macaques**
Adele Guglielmi, PhD (*University of California, Davis*)
- 14. From a Drop of Blood to a Diagnosis: Methylation-Sensitive High-Resolution Melting Unlocks Angelman Syndrome Screening in Dried Blood Spot Samples**
Leticia Guida, PhD (*Oswaldo Cruz Foundation*)

- 15. How Neurons and Brain Immune Cells Talk in Angelman Syndrome: A New Window into *UBE3A*, Inflammation, and Synapses**
Yu-Wen Alvin Huang, MD, PhD (*Brown University*)
- 16. Neuronal *UBE3A* Loss Engages a TNF-Driven Neuron-to-Microglia Axis Promoting Synaptic Engulfment in Angelman Syndrome**
Yu-Wen Alvin Huang, MD, PhD (*Brown University*)
- 17. The Angelman Syndrome Natural History Study: Key Lessons from 519 Participants**
Rachel Jardim, MPH (*Boston Children’s Hospital*)
- 18. Expanding Access to Molecular Screening to Build Clinical Trial Readiness for Angelman Syndrome in Latin America**
Melina Klurfan, MSc, JD (*FAST LatAm*)
- 19. AI-Enabled Single-Shot Design of an ALPL-Targeting AAV Capsid with Exceptional Cross-Species CNS and Muscular Delivery Efficiency Following Systemic Administration**
Jamie Kwasnieski, PhD (*Dyno Therapeutics*)
- 20. Contribution of *GABA-A* Receptor Subunit Genes to Angelman Syndrome Pathophysiology**
Eric Levine, PhD (*University of Connecticut School of Medicine*)
- 21. Exploring Angelman Syndrome Using Patient-Derived Organoids**
Emily Lien (*Children’s Mercy Kansas City*)
- 22. Antibody-Mediated Blood–Brain Barrier Modulation Enables Systemic Delivery of Antisense Oligonucleotide Treatment for Angelman Syndrome Mouse Model**
Xiaona Lu, MD, PhD (*Yale School of Medicine*)
- 23. No Genotype Left Behind: A Novel Mouse Model Recapitulating Paternal Uniparental Disomy 15 of Angelman Syndrome**
Xiaona Lu, MD, PhD (*Yale School of Medicine*)
- 24. Development of a Modular Recombinant *UBE3A* Platform for Biochemical Characterization and Non-Viral Cellular Delivery Studies**
Alejandro D. Nadra, PhD (*Universidad de Buenos Aires and CONICET*) and Celina Maria Enriz (*Universidad de Buenos Aires*)
- 25. Advancing Observer-Reported Outcome Measurement: Preliminary Quantitative Findings from the MOOD-AS**
Sarah Nelson-Potter, PhD, CCC-SLP (*RTI International*)
- 26. Investigating the Neuronal Origin of *UBE3A* in Cerebrospinal Fluid**
Morty Razavi, PhD (*SISCAPA Assay Technologies*)
- 27. Beyond the Bayley Total Score: A Novel Item-Level Framework for Cognitive Profiling in Angelman Syndrome**
Alba Richaudeau, MD, PhD (*FAST LatAm, Htal. Universitario Austral, IFIBIO UBA Conicet, Inst. Neurogenia*), Melina Klurfan, MSc, JD (*FAST LatAm*), Hernan Amartino, MD (*FAST LatAm, Htal. Universitario Austral, Inst. Neurogenia*)

- 28. Diving into the Bayley-3: Understanding Strengths and Challenges in Angelman Syndrome**
Alba Richaudeau, MD, PhD (FAST LatAm, Htal. Universitario Austral, IFIBIO UBA Conicet, Inst. Neurogenia), Melina Klurfan, MSc, JD (FAST LatAm), Hernan Amartino, MD (FAST LatAm, Htal. Universitario Austral, Inst. Neurogenia)
- 29. Stories of Angelman Syndrome Diagnosis: A Qualitative Exploration of Caregiver Lived Experience**
Abigail Ryan (Boston Children's Hospital)
- 30. Assessing Meaningful Change: Association of Functional and Behavioral Measures with Angelman Syndrome-Specific Clinical Impressions**
Anjali Sadhwani, PhD (Boston Children's Hospital)
- 31. Estimating Minimal Clinically Important Differences for Clinical Outcome Assessments in Angelman Syndrome: Towards Meaningful Endpoints for Disease-Modifying Trials**
Laurent Servais, MD, PhD (University of Oxford)
- 32. Foundation for Angelman Syndrome Therapeutics (FAST) iPSC Collection: A Patient-Derived Resource for Angelman Syndrome Research**
Rachel Steeg (Fraunhofer UK Research)
- 33. TfR1 CapX and Open Aperture: A Blood-Brain Barrier-Penetrant AAV Capsid Platform and No-Cost Academic Access Program to Accelerate CNS Gene Therapy for Angelman Syndrome and Other CNS Indications**
Andrew Steinsapir, MBA (Apertura Gene Therapy and Deerfield Management)
- 34. Development of CRISPRa Therapeutics for Angelman Syndrome**
Jiawan Sun, PhD (University of California, San Francisco)
- 35. Single IV Administration of Vectorized miRNA Enables Broad Restoration of UBE3A in the Brains of Non-Human Primates for the Treatment of Angelman Syndrome**
Veda Tatavarty, PhD (Encoded Therapeutics)
- 36. BEACON: Advancing Rugonersen into Phase 3 Development for Individuals with Angelman Syndrome**
Jorrit Tjeertes (Oak Hill Bio)
- 37. Correcting for Aperiodic Activity Enhances EEG Genotype Separation in Angelman Syndrome**
Pieter van Mierlo, PhD (Clouds of Care)
- 38. Sociodemographic Disparities in Trial Readiness and Clinical Care Among U.S. Families Affected by Angelman Syndrome**
Anne C. Wheeler, PhD (RTI International)
- 39. Expressive Communication in Angelman Syndrome: Analysis of the Data from the Angelman Syndrome Natural History Study**
Caroline Woeber, MA, CCC-SLP (Children's Hospital Colorado)

The following CURE GABA-A posters will also be featured at the poster reception:

- 40. Three Sides of the Coin: Functional Determinants of GABAA Receptor-Associated Epilepsies**
Nathan Absalom (Western Sydney University)
- 41. Gene Therapeutics for Neurodegenerative and Neurodevelopmental Diseases**
Mimoun Azzouz (University of Sheffield)
- 42. Epilepsy-Associated GABRB2 Variants Impair GABAA Receptor Biogenesis Through Distinct Proteostasis Mechanisms**
Xi (Chelsea) Chen (Case Western Reserve University School of Medicine)
- 43. An Epilepsy-Associated GABRA1 Variant Captures Neuronal Hyperexcitability in a Human Induced Pluripotent Stem Cell Model**
Bianca de Freitas Brenha (Case Western Reserve University School of Medicine)
- 44. Development of a GABRB3 Patient NAM for Drug Screening**
Angela Duong (NeuCyte Inc.)
- 45. GABRB3 Mutations Impair GABAA Receptor Trafficking and Synaptic Localization in Epilepsy**
Jing-Qiong (Katty) Kang (Vanderbilt University Medical Center)
- 46. Electrophysiological Biomarkers of GABA Metabolism in Children with Genetic Epilepsy Syndromes**
Sakar Rijal (Cook Children's Health Care System)
- 47. Exploring GABAA Receptor Function in Neurological Disorders: Insights from Xenopus Oocyte Microtransplantation**
Cristina Roseti (University of Insubria)
- 48. Investigating Proteostasis Disruption in GABRA1 Frameshift Variants and its Implications for Epilepsy Pathogenesis**
Marnie Williams (Case Western Reserve University School of Medicine)

Have questions?

**Wondering how everything you learned
applies to your loved one?**

If you reside in the USA, you can schedule a 1:1 meeting
with FAST's certified genetic counselor.



Scan the QR code
below to make an
appointment with
Niki Armstrong, MS, CGC.



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.	ube-cel: HSC Gene Therapy Program Update	Allyson Berent, DVM, DACVIM <i>AS²Bio and TransformaTx Bio</i>
9:20 a.m.	MVX-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> Allyson Berent, DVM, DACVIM <i>AS²Bio and CourageAS</i> Elizabeth Berry-Kravis, MD, PhD <i>Rush University Medical Center</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.	GTX-102 Clinical Program Overview	Emil Kakkis, MD, PhD <i>Ultragenyx</i>
12:20 p.m.	Panel Discussion: Questions from the Community	Allyson Berent, DVM, DACVIM

Partners in Pursuit of a Cure

Your generosity fuels every breakthrough, every moment of connection, and every step toward a brighter future for individuals living with Angelman syndrome. Thank you for believing in this mission alongside us.

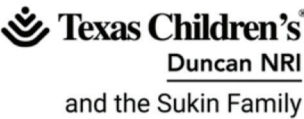
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