

# Friday, December 2

## Focus on Angelman Syndrome

### Translational Research

Time	Speaker/Organization	Topic
8:30 am	<b>Craig Martin</b> Global Genes	<i>Breaking the Mold: How Patient Groups like FAST are Reshaping Drug Development in Rare Disease</i>
9:20 am	<b>Allyson Berent, DVM, DACVIM</b> FAST	<i>FAST Roadmap to a C.U.R.E. 2.0</i>
10:05 am	Break	
10:20 am	<b>Sharyl Fyffe-Maricich</b> Ultragenyx	<i>Gene Therapy for Rare Genetic Neurodevelopmental Disorders: The Basics</i>
11:10 am	<b>Albert J. Keung, PhD, Amay Bandodkar, PhD, Z. Begum Yagci, R. Chris Estridge, Tyler Johnson and Navya Mishra</b> North Carolina State University	<i>Advancing Human Stem Cell-Derived Platforms for Angelman Syndrome Research</i>
11:35 am	<b>Scott Dindot, PhD</b> Texas A&M College of Medicine	<i>Angelman Syndrome Pig Model: Characterization and Future Directions</i>
11:50 am	<b>Mei Baker, MD</b> University of Wisconsin School of Medicine and Public Health  <b>Katerina (Kate) S. Kucera</b> RTI (Research Triangle Institute) International	<i>Development and Validation of a Newborn Screening Test for Angelman Syndrome</i>  <i>Toward Universal Newborn Screening for Angelman Syndrome: The Early Check Approach</i>
12:15 pm	<b>Sarah Pitluck</b> SP Consulting, LLC  <b>John Jarvis</b> Medicus Economics	<i>The Importance of Patient Input for Coverage and Payment of New Therapies</i>
12:30 pm	Lunch	
1:15 pm	<b>Allyson Berent, DVM, DACVIM</b> FAST	<i>Angelman Syndrome Biomarker and Outcome Measure Consortium: What's the Hype? Why Does it Matter So Much?</i>
1:50 pm	<b>Christina K. Zigler, PhD, MEd</b> Duke University School of Medicine	<i>A Family-Centered Approach to Measuring Communication Ability within Clinical Trials</i>
2:05 pm	<b>Robert Carson, MD, PhD</b> Vanderbilt Brain Institute	<i>Crowd-Sourcing Research into Nonepileptic Myoclonus in Angelman Syndrome</i>

2:15 pm	<b>Meagan Cross</b> FAST Australia <b>Isabel Orellana de Chang</b> FAST LatAm <b>Amelia Beatty</b> FAST USA	<i>FAST Global Search &amp; Rescue Initiative: Finding Every Individual Globally Living with Angelman Syndrome and Why it Matters</i>
2:40 pm	<b>Amelia Beatty</b> FAST USA <b>Stephanie Azout</b> FAST LatAm <b>David Fernández</b> FAST Spain <b>Charlotte Préstat</b> FAST France <b>Benedetta Sirtori</b> FAST Italy <b>Tom Keogh</b> FAST UK <b>Noah Firestone</b> FAST Canada <b>Meagan Cross</b> FAST Australia	<i>Update on Progress Around the World from FAST Global</i>
3:05 pm	Break	
3:20 pm	<b>James M. Wilson, MD, PhD</b> Perelman School of Medicine, University of Pennsylvania	<i>hUBE3 A-AAV9 Gene Replacement Therapy for Angelman Syndrome: Progress Toward the Clinic</i>
3:55 pm	<b>Yong-Hui Jiang, MD, PhD and Jianbing Zhou, PhD</b> Yale School of Medicine	<i>Novel Gene Editing Approach for Long-Term Paternal Gene Activation</i>
4:20 pm	<b>Alana Newhouse</b> FAST <b>Ryan Jacob</b> FAST	<i>Fireside Chat: The FAST Commercial Philosophy</i>
4:45 pm	<b>All Presenters</b>	<i>Panel Discussion and Audience Q&amp;A</i>
5:30 pm	<b>Allyson Berent, DVM, DACVIM</b> FAST	<i>Closing Remarks</i>

# Saturday, December 3

## Focus on Clinical Trials

Time	Speaker/Organization	Topic
8:30 am	<b>Wendy Chung, MD, PhD</b> Columbia University	<i>Rapidly Evolving Opportunities for Treatments for Rare Genetic Diseases</i>
9:15 am	<b>Jennifer Panagoulis, RAC</b> FAST, Angelman Syndrome Biomarker and Outcome Measure Consortium	<i>Clinical Trial Basics: What Parents Need to Know About Trial Participation</i>
9:45 am	<b>Emil Kakkis, MD, PhD</b> Ultragenyx	<i>The Development of Rare Disease Therapeutics: Compassion and Transparency</i>
10:15 am	<b>Rebecca Crean, PhD</b> Ionis Pharmaceuticals, Inc.	<i>An Update on HALOS Clinical Trial in Individuals with Angelman Syndrome</i>
10:45 am	<b>Brenda Vincenzi, MD</b> Roche Pharmaceuticals	<i>Roche Angelman Syndrome Program Update</i>
11:15 am	Break	
11:30 am	<b>Jennifer Panagoulis, RAC</b> Transformatx Biotherapeutics	<i>Hematopoietic Stem Cell Gene Therapy: What is ube-cel?</i>
11:45 pm	<b>Nancy E. Jones, PhD</b> Neuren Pharmaceuticals	<i>NNZ-2591 as a Treatment for Angelman Syndrome</i>
12:05 pm	<b>Stephanie Ciarlone, PhD</b> PTC Therapeutics	<i>PTC-AS Gene Therapy Program Update</i>
12:25 pm	<b>All Presenters</b>	<i>Panel Discussion and Audience Q&amp;A</i>
1:15 pm	<b>Allyson Berent, DVM, DACVIM</b> FAST	<i>Closing Remarks</i>