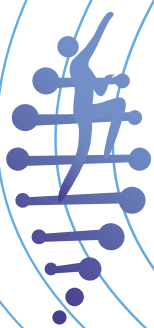


**15th Annual  
Global Science Summit  
Program**

**fast**



**Dream**

**Big.**

**Friday, December 2, 2022**

**8:30 am – 5:30 pm**

**Global Science Summit:  
Focus on Angelman Syndrome  
Translational Research**

**Saturday, December 3, 2022**

**8:30 am – 1:15 pm**

**Global Science Summit:  
Focus on Clinical Trials**



### **Caregiver Impact Survey**

Angelman syndrome caregivers, we need you!  
In coordination with Medicus Economics, we are conducting a Caregiver Impact Survey. The data collected will demonstrate the impact of AS caregiving in a way that previous research hasn't captured. This is critical information that insurance companies and governments will need to cover the cost of therapeutics. Plus, this study will help communicate the benefits of AS therapies to policymakers, government agencies and others who provide financial assistance to AS families.

Please visit our booth to start the survey.

### **Event Website**

For more information, including speaker bios and a list of exhibitors, visit the Summit & Gala website by scanning the QR code above.

### **WiFi Instructions**

To access WiFi within the event space, please use the following network and password:  
Network: FASTSummitGala  
Passcode: dreambig

# 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,</b> <b>Amay Bandodkar, PhD,</b> <b>Z. Begum Yagci, R. Chris Estridge,</b> <b>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>





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**Dream Big.**

**We DREAM to CURE AS!**

*Love, Quincy, Vivian, Sydney, Ryder, Theo & all of us at Quincy's Quest*



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We proudly support the Angelman syndrome community. Your tenacity motivates us to work every day to find a treatment. Together we can **boldly** transform life with Angelman syndrome.

Thank you, families, for including Ovid in this important event. We are so happy to be back together in person.




**Celebrating yes**

And our commitment to delivering breakthrough medicines to patients



**Improving the Lives of People with Neurodevelopmental Disabilities**

Neuren is developing new therapies for debilitating neurodevelopmental disorders emerging in early childhood, characterised by impaired connections and signalling between brain cells. The first, for Rett syndrome, is under Priority Review by FDA with a target action date in March 2023.

**2 novel drugs, targeting 6 disorders, all with Orphan Drug designation**

NNZ-2591 is in Phase 2 development targeting four syndromes including Angelman

Neuren is currently enrolling a **Phase 2 clinical study in Angelman syndrome** at Brisbane, Sydney and Melbourne in Australia


Neuren Pharmaceuticals Limited (ASX: NEU), Suite 201, 697 Burke Road, Camberwell, VIC 3124, Australia

**Rare diseases, real strides to treat them—this is why we're here.**

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**Ulysses Neuroscience Ltd.** is an Irish SME aimed at advancing knowledge and treatment of brain disorders by providing clinical and preclinical research services to pharmaceutical companies to accelerate their drug discovery programmes in neuropsychiatric, rare neurodevelopmental and neurodegenerative disorders.

We are committed to re-incorporating humanity into all aspects of how we do science. The company is based on three core values which directly drive our research and interaction with pharmaceutical companies: patient-centricity; translational research and social responsibility. Our core values aim to lead a new journey against brain disorders which will eventually result in the realisation of the revolutionary concept of discovering new treatments "with the patients".



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