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.

Dream Big.

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	**Craig Martin** Global Genes	*Breaking the Mold: How Patient Groups like FAST are Reshaping Drug Development in Rare Disease*
9:20 am	**Allyson Berent, DVM, DACVIM** FAST	*FAST Roadmap to a C.U.R.E. 2.0*
10:05 am	Break	
10:20 am	**Sharyl Fyffe-Maricich** Ultragenyx	*Gene Therapy for Rare Genetic Neurodevelopmental Disorders: The Basics*
11:10 am	**Albert J. Keung, PhD, Amay Bandodkar, PhD, Z. Begum Yagci, R. Chris Estridge, Tyler Johnson and Navya Mishra** North Carolina State University	*Advancing Human Stem Cell-Derived Platforms for Angelman Syndrome Research*
11:35 am	**Scott Dindot, PhD** Texas A&M College of Medicine	*Angelman Syndrome Pig Model: Characterization and Future Directions*
11:50 am	**Mei Baker, MD** University of Wisconsin School of Medicine and Public Health	*Development and Validation of a Newborn Screening Test for Angelman Syndrome*
	Katerina (Kate) S. Kucera RTI (Research Triangle Institute) International	*Toward Universal Newborn Screening for Angelman Syndrome: The Early Check Approach*
12:15 pm	**Sarah Pitluck** SP Consulting, LLC **John Jarvis** Medicus Economics	*The Importance of Patient Input for Coverage and Payment of New Therapies*
12:30 pm	Lunch	
1:15 pm	**Allyson Berent, DVM, DACVIM** FAST	*Angelman Syndrome Biomarker and Outcome Measure Consortium: What's the Hype? Why Does it Matter So Much?*
1:50 pm	**Christina K. Zigler, PhD, MSEd** Duke University School of Medicine	*A Family-Centered Approach to Measuring Communication Ability within Clinical Trials*
2:05 pm	**Robert Carson, MD, PhD** Vanderbilt Brain Institute	*Crowd-Sourcing Research into Nonepileptic Myoclunus in Angelman Syndrome*
2:15 pm	**Meagan Cross** FAST Australia **Isabel Orellana de Chang** FAST LatAm **Amelia Beatty** FAST USA	*FAST Global Search & Rescue Initiative: Finding Every Individual Globally Living with Angelman Syndrome and Why it Matters*
2:40 pm	**Amelia Beatty** FAST USA **Stephanie Azout** FAST LatAm **David Fernández** FAST Spain **Charlotte Préstat** FAST France **Benedetta Sirtori** FAST Italy **Tom Keogh** FAST UK **Noah Firestone** FAST Canada **Meagan Cross** FAST Australia	*Update on Progress Around the World from FAST Global*
3:05 pm	Break	
3:20 pm	**James M. Wilson, MD, PhD** Perelman School of Medicine, University of Pennsylvania	*hUBE3 A-AAV9 Gene Replacement Therapy for Angelman Syndrome: Progress Toward the Clinic*
3:55 pm	**Yong-Hui Jiang, MD, PhD and Jianbing Zhou, PhD** Yale School of Medicine	*Novel Gene Editing Approach for Long-Term Paternal Gene Activation*
4:20 pm	**Alana Newhouse** FAST **Ryan Jacob** FAST	*Fireside Chat: The FAST Commercial Philosophy*
4:45 pm	**All Presenters**	*Panel Discussion and Audience Q&A*
5:30 pm	**Allyson Berent, DVM, DACVIM** FAST	*Closing Remarks*

Saturday, December 3
Focus on Clinical Trials

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

Notes

Strength.

You might not see it—strength comes from within. It is not always evident in your abilities. It is revealed by overcoming the insurmountable.

We are inspired by the courageous angels and their families who prove their strength everyday.

Thanks to your generous support of FAST, new innovations are making the impossible inevitable.

Make a donation today
www.cureangelman.org/donate

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