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virtually
UNSTOPPABLE

2020 FAST GLOBAL SUMMIT & MINI-GALA

DEC
4-5

CURE
ANGELMAN.
LIVE

2020 FAST VIRTUAL GLOBAL SUMMIT AND MINI-GALA SCHEDULE OF EVENTS

Register: cureangelman.live

Friday, December 4, 2020

9:00 AM – 5:30PM CST*

Science Summit

Visit our vendors and sponsors throughout the day – located in the Expo Hall

9:00-9:30 AM

KEYNOTE SPEAKER

David Meeker, M.D., Chairman, President and Chief Executive Officer, Rhythm Pharmaceuticals, Inc.

The Patient's Role in Drug Development

9:30-10:20 AM

Allyson Berent, DVM, DACVIM, Chief Science Officer, FAST

Overview of Therapeutic Strategies for the Treatment of Angelman Syndrome

10:20-10:30 AM

Break

10:30 -10:52 AM

Bryce Reeve, Ph.D., Duke University

Validation of the Observer-Reported Communication Ability (ORCA) Measure in Individuals with Angelman Syndrome

10:52-11:14 AM

Yong-Hui Jiang, M.D., Ph.D., Yale University

Angelman IPSC and Brain Organoid Biorepository at Yale

11:14-11:37 AM

Albert Keung, Ph.D., North Carolina State University

Human Cerebral Organoids: Benefits and Challenges for Angelman Syndrome Research

11:37-11:57 PM

Bruce Bloom, Ph.D., Chief Collaboration Officer, Healx

Using AI and Repurposing to Predict and Develop New Therapies for Rare Diseases

11:57-12:45 PM

Lunch

12:45-1:12 PM

Emil Kakkis, M.D., Ph.D., CEO, President and Founder, Ultragenyx Pharmaceutical, Inc.

Learnings Guiding Clinical Trial Design for Heterogenous Populations

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- 1:12-1:33 PM Suyash Prasad, MBBS, M.Sc., MRCP, MRCPCH, FFPM, Chief Medical Officer, Head of R&D; Emily McGinnis, Chief Patient Officer, Head of Government Affairs, Taysha Gene Therapies
Putting Patients at the Center: An Overview of Taysha and Our Gene Therapy Development Process
- 1:33-1:48 PM Larry Glass, Neuren Pharmaceuticals
Background on Neuren Pharmaceuticals and NNZ-2591
- 1:48-1:59 PM Stephanie Ciarlone, Ph.D., PTC Therapeutics
Gene Therapy in Angelman Syndrome
- 1:59-2:22 PM Jim Wilson, M.D., Ph.D., University of Pennsylvania
Update on Gene Therapy for CNS Diseases
- 2:22-2:28 PM Joe Anderson, Ph.D., University of California, Davis
Hematopoietic Stem Cell Gene Therapy as a Treatment Strategy for Angelman Syndrome: IND-Enabling Status Update
- 2:28-2:38 PM BREAK
- 2:38-2:58 PM Becky Crean Ph.D., Ionis Pharmaceuticals
Updates on the Development of an Antisense Oligonucleotide Therapy for Angelman Syndrome
- 2:58-3:19 PM Rob Komorowski, Ph.D., J.P. Gilbert, Ph.D., Biogen
Accelerating Clinical Development Capabilities in Angelman Syndrome: Update on CSF and EEG Based Biomarkers
- 3:19-3:41 PM Brenda Vincenzi, M.D., Roche Pharmaceuticals
Roche Angelman Syndrome Program Update to the FAST Community
- 3:41-4:06 PM Scott Stromatt, M.D., GeneTx Biotherapeutics; Elizabeth M. Berry-Kravis, M.D., Ph.D., Co-Director, Molecular Diagnostics Section of The Genetic Laboratory, Professor, Dept. of Pediatrics, Rush Medical College
The Use of GTX-102, an Antisense Oligonucleotide for Paternal UBE3A Gene Activation, as a Potential Therapeutic for Treating Individuals Living with Angelman Syndrome
- 4:06-4:16 PM Jeremy M. Levin, D.Phil, MB BChir, Chairman, CEO, Ovid Therapeutics
Angelman Syndrome-2020 A Turning Point; The NEPTUNE Phase 3 Clinical Trial
- 4:16-4:20 PM BREAK
- 4:20-5:20 PM Round table discussion – ask your questions!
Parent/Caretaker Q&A with Pharma/Biotech Companies, Researchers, and Clinicians

**Times may adjust and schedule will be updated accordingly*

Friday, December 4, 2020
Immediately Following the Science Summit
Cocktail Hour @ Big Bar

Saturday, December 5, 2020
8:00 AM – 4 PM CST
Translational Research Symposium
Angelman Syndrome Biomarker and Outcome Measure Meeting
By Invitation Only
Please contact allyson.berent@cureangelman.org for more information

Saturday, December 5, 2020
9:00 AM – 11:45 PM CST*
Educational Summit

Visit our vendors and sponsors throughout the day – located in the Expo Hall

	Track A	Track B
9-9:45	Homeschooling During Covid19+ Virginia Spencer, M. Ed. Lumen Advocacy LLC	Beyond the Clinic: Enabling Function Through Guerilla OT+ Kelly Beins OTR/L SI(c)
9:45-10:30	Toilet Training in Individuals with Angelman Syndrome+ Terry Katz Ph.D.	From Grandiose to Real Life- How to Embed Communication Into Your Everyday Routines+ Lauren DeValk, MOTR/L Caroline Woeber, MA, CCC-SLP
10:30-11:15	Approach to Sleep Challenges in AS+ Jessica Duis MD Terry Katz Ph.D.	Recognizing and Preventing Caregiver Burnout+ Amanda Simmons, MS, OTR/L
11:15-11:45	Adult Transition Services Tracy Carreola Juli DelMonego	Telehealth- What to Expect+ Lauren DeValk, MOTR/L Jessica Duis, MD, MS Wendy Pierce, MD Caroline Woeber, MA, CCC-SLP

**Times may adjust and schedule will be updated accordingly. +Live Q&A via Zoom to follow session.*

Saturday, December 5, 2020

6:00 PM CST

FAST mini-Gala

Welcome to the 2020 FAST Virtually Unstoppable mini-Gala

Guest of Honor: Colin Farrell

Gala Video

Virtual Paddle Raise

Entertainment by 7th Heaven

Get Social Tables

Saturday night's *Virtually Unstoppable* mini-Gala will include the memorable moments that the FAST community appreciates each year. Paula Evans and Allyson Berent will speak, celebrating our community's great achievements this year. Colin Farrell, AS dad and guest of honor, will make a special appearance, charming the audience with his usual wit and humor. The Gala Video, a yearly tradition, will highlight our children and their numerous accomplishments. The FAST community is always so generous so we couldn't leave out a virtual paddle raise. 7th Heaven's entertainment will make you want to get up and dance, even in your living room. Afterwards, join your friends to experience the Virtually Unstoppable Gala, live and (almost) in person!

FAST

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