

Agenda

MONDAY, JUNE 5TH — MOLECULAR AND CELLULAR FUNCTIONS OF MECP2

10:00 am Welcome

SESSION 1: MECP2 BASIC AND SURPRISING FUNCTIONS

10:10 am **Keynote: Dr. Rudolf Jaenisch**
MECP2, Rett Syndrome, and Gene Expression

10:40 am **Dr. Yechezkel Sztainberg**
Antisense Oligonucleotide Therapy for the MECP2 Duplication Syndrome: A Preclinical Study

11:00 am **Dr. Michael Müller**
Deciphering the Mitochondrial Impairment in Rett Syndrome

11:20 am **Dr. Sameer Bajikar**
Timed Molecular, Behavioral, and Physiological Characterization of Mice After Adult Depletion of MeCP2 (Poster #2)

11:40 am **Dr. Jing Zhang**
Advancing an Understanding of MeCP2 Function by Identification of Methylated RNA Targets (Poster #52)

12:00 pm **Poster Flash Talks (#1-#26)**

12:30 pm **LUNCH**

1:15 pm **Dr. Ponni Subbiah, Acadia Pharmaceuticals**
Presenting Sponsor Presentation

SESSION 2: CONTROL OF MECP2 (OVER)EXPRESSION

1:25 pm **Keynote: Dr. Huda Zoghbi**
Rett Syndrome and MECP2 Disorders: An Enlightening Dialogue Between Bench and Clinic

1:55 pm **Dr. Hume Stroud**
Exploring the Formation and Function of Novel MECP2 Genomic Binding Sites

2:15 pm **Dr. Vania Broccoli**
Improved Tools and Methods for MeCP2 Gene Replacement Therapy in Rett Syndrome

2:35 pm **Dr. James Ellis**
Buffering of Transcription Rate by mRNA Half-Life in Rett Syndrome Neurons and In Vitro Neurodevelopment

2:55 pm **Dr. Lisa Boxer**
Investigating the Immediate Consequences of Acute Loss of MeCP2 (Poster #5)

3:15 pm **BREAK**

SESSION 3: MECP2 OUTSIDE OF ITS COMFORT ZONE

3:35 pm **Keynote: Dr. Nathaniel Heintz**
5hmC, MeCP2 and Epigenetic Control of Neuronal Function

4:05 pm **Dr. Nicolas Panayotis**
Importins: A New Target for Ameliorating Neuronal Functions in MeCP2-Related Disorders

4:25 pm **Dr. Janine LaSalle**
Sex Disparate Gut Microbiome and Metabolome Perturbations Precede Disease Progression in a Mouse Model of Rett Syndrome

4:45 pm **Dr. Rocco Gogliotti**
Insights from a Reverse Translational Approach to Rett Syndrome Target Identification

5:05 pm **Ms. Gabriella Chua**
Single-molecule Visualization of the Behavior of MeCP2 on Chromatin (Poster #9)

5:25 pm **Poster Flash Talks (#27-#52)**

6-7:30 pm **Poster Session/Reception**

Agenda

TUESDAY, JUNE 6TH - THERAPEUTIC APPROACHES FOR RETT SYNDROME

- 7:00 am **BREAKFAST**
8:00 am **Welcome**
8:10 am **Panel Discussion: Day 1 Keynote Speakers**
9:10 am **Late-Breaking Poster Flash Talks** (see supplemental handout)
9:50 am **BREAK**

SESSION 4: INSIGHTS FROM AFFECTED FAMILIES & OTHER DISORDERS

- 10:00 am **Keynote: Dr. Katherine High**
Gene Therapy for CNS Disorders: Successes, Challenges, and Opportunities
10:30 am **Dr. Darcy Krueger**
Tuberous Sclerosis Complex (TSC): Is Prevention in a Rare Genetic Condition Possible?
10:50 am **Dr. Richard Finkel**
Developing Effective Disease-Specific Outcome Measures for a Rare Disease: The Example of Spinal Muscular Atrophy
11:10 am **Ms. Keri Ramsey**
Family and Caregiver Perspectives on Gene Therapy for Rett Syndrome (Poster #44)
11:30 am **Caregiver Panel: Living with Rett Syndrome**
12:10 pm **LUNCH**

SESSION 5: INNOVATIVE THERAPIES

- 1:10 pm **Keynote: Dr. Jeannie Lee**
Advancing X Reactivation as a Method of Treating Rett Syndrome
1:40 pm **Dr. Ronald Emeson**
RNA Editing-Mediated Modification of Common Nonsense Mutations in Rett Syndrome
2:00 pm **Dr. Yuval Tabach**
Expanding the MECP2 Network Using Comparative Genomics Reveals Therapeutic Options for Rett Syndrome
2:20 pm **Dr. Julian Halmai**
A Miniature Cas9 for Epigenetic Editing in Rett Syndrome (Poster #24)
2:40 pm **Dr. Andrew Dietz**
Massive Diversity Capsid Screening and Machine Learning Identify Next-Generation AAV for Targeted Tissue Biodistribution (Poster #14)
3:00 pm **BREAK**

SESSION 6: OUTCOME MEASURES AND BIOMARKERS

- 3:30 pm **Keynote: Dr. Michelle Campbell**
How Data Informs Drug Development
4:00 pm **Dr. Michela Fagiolini**
Therapeutic Targeting of the Choroid Plexus in Rett Syndrome
4:20 pm **Dr. Victor Faundez**
Rett Syndrome Biomarker Discovery Through Systems Biology
4:40 pm **Dr. Jonathan Merritt**
A Predictive Model of Clinical Severity in Rett Syndrome (Poster #34)
5-6:30 pm **Poster Session**
6:30-8 pm **Center of Excellence Director Dinner in Crescent Ballroom (By Invite Only)**

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WEDNESDAY, JUNE 7TH — TREATMENTS ON THE HORIZON FOR RETT SYNDROME

- 7:00 am** **BREAKFAST**
- 8:00 am** **Welcome**
- 8:05 am** **Keynote: Dr. James Youakim**
Development of Trofinetide for Rett Syndrome: From Phase 2 Studies to Launching DAYBUE™
- 8:55 am** **Dr. Benit Maru and Ms. Emily McGinnis**
Taysha's Investigational Gene Therapy Program Update
- 9:15 am** **Dr. Stuart Cobb and Dr. Albenia Patroneva**
NGN-401: A Novel Regulated Gene Therapy for Rett Syndrome
- 9:35 am** **Dr. Carolyn Ellaway**
NTI164 | A Novel, Full-Spectrum Medicinal Cannabis Derived Treatment for Rett Syndrome
- 9:55 am** **BREAK**
- 10:15 am** **Dr. Jeff Neul**
Novel Study Design: Umbrella Protocol for Repurposed Drugs
- 10:30 am** **Dr. Kathrin Meyer**
Reactivation of the Silent X Chromosome via Gene Therapy is Efficacious in Rett and Other X-linked Disorders (Poster #35)
- 10:45 am** **Dr. Frederic Vigneault**
Discovery and Development of a Novel Broad-Acting Rett Syndrome Therapeutic Candidate (Poster #50)
- 11:00 am** **Dr. Nicholas Tonks**
PTP1B Inhibition — A Mechanism-Based Approach to the Treatment of Rett Syndrome
- 11:15 am** **Panel Session: Highlights & Next Steps**
- 12:00 pm** **MEETING CONCLUDES**
(Box lunch to be provided)