

AGENDA

(All times: Central Time, United States)
All Sessions and Meals in Ryman Ballroom

TUESDAY, APRIL 26

- 12:00 pm **Registration**
- 1:00 pm **Welcome** – Dominique Pichard, MD (International Rett Syndrome Foundation)
- 1:05 pm **Keynote Lecture: MeCP2 as a microsatellite DNA binding protein** –
Christophe Papin, PhD (IGBMC)
- 1:35 pm **Oral Presentations**
- **Single-molecule visualization of the interaction between MeCP2 and chromatin** – Gabriella Chua (The Rockefeller University)
 - **Pathophysiological length variation, structure, and phase separation of polyA repeats in the NTD and TRD of MeCP2** – Ferdinando Fiumara, MD, PhD (University of Torino)
 - **Transcriptomic insights into the etiology of Rett syndrome** – Daniel Connolly (University of Pennsylvania)
- 2:35 pm **Poster Flash Talks**
- 2:55 pm **BREAK**
- 3:15 pm **Keynote Lecture: X-chromosome reactivation** –
Vincent Pasque, PhD (University of Leuven)
- 3:45 pm **Oral Presentations**
- **Use of ultrasound-based technology to optimize brain gene therapy treatment of mouse models of Rett syndrome** – Jean-Christophe Roux, PhD (Aix Marseille Université)
 - **Non-invasive interventions may synergistically enhance MECP2 gene therapy** – Sarah Sinnott, PhD (University of Texas Southwestern Medical Center)
 - **Safety and Biodistribution Assessment in Non-human Primates (NHPs) of a miniMECP2 AAV9 Vector for Gene-replacement Therapy of Rett Syndrome** – Suyash Prasad (Taysha Gene Therapies)
- 4:45 pm **Poster Flash Talks**
- 5:00 pm - **Poster Session and Reception – Ryman Ballroom AB Foyer**
6:30 pm
- 6:30 pm **Dinner on your own**
- 7:30 pm - **Breakout Sessions:**
9:00 pm 1) What does MeCP2 do in the nucleus?
 2) What readouts for high throughput screening are useful in Rett?
 3) What is sufficient preclinical evidence in order to move to a clinical trial?

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WEDNESDAY, APRIL 27

7:00 am Continental Breakfast

8:00 am Welcome

8:05 am **Keynote Lecture: Using human organoids to understand aspects of Rett syndrome -**
Bennett Novitch, PhD (UCLA)

8:35 am **Oral Presentations**

- **A high throughput screen platform for studying Rett syndrome -**
Qiang Chang, PhD (University of Wisconsin-Madison)
- **L1-mediated inflammation in Rett syndrome -** Alysson Muotri, PhD (UCSD)
- **Mitochondrial dynamics and astrocyte contribution to development of Rett pathophysiology in human ESC derived cerebral organoids -** Danielle Tomasello, PhD (Whitehead Institute for Biomedical Research)
- **Overview of rodent models of Rett syndrome -** Rodney Samaco, PhD (Baylor College of Medicine)
- **A comparative longitudinal study of Magnetic Resonance Imaging in the brain of different Mecp2 deficient mouse models sheds some light on the brain pathology and confirms the limit of using a single mouse model in pre-clinical studies -**
Nicoletta Landsberger, PhD (University of Milan)

10:15 am **BREAK**

10:40 am **Keynote Lecture: Evoked potentials in mouse models and humans -**
Eric Marsh, MD, PhD (CHOP Research Institute)

11:10 am **Oral Presentations**

- **Altered adaptation of somatosensory evoked activity reveals deficits in cortical plasticity in a mouse model of Rett syndrome -** Kerry Delaney, PhD (University of Victoria)
- **Evaluation of the therapeutic potential of ketamine in a mouse model of Rett syndrome -**
Michelle Piazza (Vanderbilt University)
- **Exploration of group II metabotropic glutamate receptor modulation in mouse models of Rett syndrome and MECP2 Duplication syndrome -** Colleen Niswender, PhD (Vanderbilt University)
- **Potentiation of mGluR(7) rescues neurophysiological features in a mouse model of Rett Syndrome -** Hongwei Dong, PhD (Vanderbilt University Medical Center)

12:30 pm Lunch (Box lunch provided)

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- 1:30 pm **Keynote Lecture: Natural History Study -**
Jeff Neul, MD, PhD (Vanderbilt Kennedy Center)
- 2:00 pm **Oral Presentations**
- **A Psychometric Evaluation of the Rett Syndrome Behaviour Questionnaire in children and adults** - Walter Kaufmann, MD (Anavex Life Sciences Corp.)
 - **Rett syndrome in adulthood: the caregiver perspective** - Kristin Phillips (Taysha Gene Therapies)
- 2:40 pm **BREAK**
- 3:00 pm **Keynote Lecture: Gait Analysis in Rett syndrome -**
Bernhard Suter, MD (Baylor College of Medicine)
- 3:30 pm **Oral Presentations**
- **Rett Syndrome: Quantifying gross motor skills and the effect of physical therapy** - Briana Czerwinski, DPT (Kennedy Krieger Institute)
 - **Efficacy and safety of trofinetide for the treatment of Rett syndrome: results from the pivotal phase 3 LAVENDER study** - Jeff Neul, MD, PhD (Vanderbilt Kennedy Center)
- 4:10 pm **Breakout Session Readouts**
- 5:00 pm - 7:00 pm **Adjourn - Cocktail Reception with Families at Water's Edge in Delta Atrium**