



IRSF RETT SYNDROME SCIENTIFIC MEETING: **2022**
ACCELERATING DISCOVERIES
IN RETT RESEARCH

PRESENTED BY

TAYSHA
GENE THERAPIES

HOSTED BY

 **International
Rett Syndrome
Foundation**

AGENDA

(All times: Central Time, United States)

TUESDAY, APRIL 26

- 12:00 pm **Registration**
- 1:00 pm **Welcome**
- 1:05 pm **Keynote Lecture: MeCP2 as a microsatellite DNA binding protein -**
Ali Hamiche, 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)
 - **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)
 - **Non-invasive interventions may synergistically enhance MECP2 gene therapy -** Sarah Sinnott, PhD (University of Texas Southwestern Medical Center)
- 4:45 pm **Poster Flash Talks**
- 5:00-6:30 pm **Poster Session and Reception**
- 7:30-9:00 pm **Breakout Sessions**

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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 -**
Bennet 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 -** Daniella Tomasello, PhD (Whitehead Institute for Biomedical Research)
- **Overview of rodent models -** 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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WEDNESDAY, APRIL 27

- 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 (Taysa 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 Read Outs**
- 5:00 pm** **Adjourn – Cocktail Reception with Families**