



**It's time to register your place for the Virtual Global Research and Trial Update**

**Saturday 13<sup>th</sup> April 2024**

## **PROGRAMME**

### **2:00-2:35 pm Neurodegeneration in Wolfram Syndrome: casting a wider net!**

Dr Nayana Gaur – PhD. University of Tartu, Laboratory Animal Centre. Tartu, Estonia

Registration link: [https://us02web.zoom.us/webinar/register/WN\\_tEa5bW1iRUOQoheeUNgo5Q](https://us02web.zoom.us/webinar/register/WN_tEa5bW1iRUOQoheeUNgo5Q)

### **2:45-3:20 pm Unified Care for Diverse Challenges: Crafting National Guidelines for Wolfram Syndrome from Diagnosis to Care**

Dr Giulio Frontino and Dr Maurizio Delvecchio, IRCCS San Raffaele Hospital, Milan, Italy

Registration link: [https://us02web.zoom.us/webinar/register/WN\\_-Y1FKqSzT1ag8sG7MAj-Ng](https://us02web.zoom.us/webinar/register/WN_-Y1FKqSzT1ag8sG7MAj-Ng)

### **3:30-4:05pm TREATWolfram Trial Update**

**Prof Timothy Barrett**

Leonard Parsons Professor of Paediatrics, NIHR Senior Investigator. Director, Centre for Rare Disease Studies. Institute of Cancer and Genomic Sciences - University of Birmingham.

Honorary Consultant Paediatric Endocrinology and Diabetes - Birmingham Women's and Children's Hospital NHS Trust

Registration link: [https://us02web.zoom.us/webinar/register/WN\\_w8WaGF\\_sSxiyYY1pcnGdew](https://us02web.zoom.us/webinar/register/WN_w8WaGF_sSxiyYY1pcnGdew)

### **4:15-4:50pm Diagnosis, Management, and Current and Future Clinical Trials for Wolfram syndrome.**

Fumihiko Urano, MD, PhD, Samuel E. Schechter Professor of Medicine, Washington University School of Medicine, St. Louis, USA

### **Preliminary Results From A Phase 2, Open-Label Study Evaluating an Oral, Fixed-Dose Combination of Sodium Phenylbutyrate and Taurursodiol in Wolfram Syndrome (HELIOS)**

Amylyx Pharmaceuticals and Fumihiko Urano, MD, PhD, Washington University

Registration link: [https://us02web.zoom.us/webinar/register/WN\\_6o1lcLlqRrW-yKb1o8ml2w](https://us02web.zoom.us/webinar/register/WN_6o1lcLlqRrW-yKb1o8ml2w)