PROVISIONAL SCIENTIFIC PROGRAM

Thursday 12 November 2020

- **Session 1: Clinical studies in MPS III: gene therapy**

9:30 **Prof. Rob Wynn, Blood and Marrow Transplant Unit, Royal Manchester Children's Hospital and University of Manchester, UK**
Keynote presentation - Report of the first ever lentiviral vector, gene-modified stem cell transplant in a patient with MPS IIIA

10:00 **Dr. Simon Jones, Willink Unit, Manchester Centre for Genomic Medicine, St Mary Hospital, Manchester Academic Health Sciences Centre, University of Manchester, UK**
Clinical trial design for treatment of Mucopolysaccharidosis Type III

10:30 **Dr Samantha Parker, Lysogene, Paris, France**
Lysogene gene therapy program for MPS IIIA

11:00 **Abeona Therapeutics, Dallas, Texas, USA *(Speaker TBC)***
*Interim results of the Transpher A phase 1/2 clinical trial of ABO-102 gene therapy for Sanfilippo syndrome type A and/or - Interim results from Transpher B, phase 1/2 clinical trial of ABO-101 gene therapy for Sanfilippo syndrome type B (***Title TBC)**

- **Session 2: Clinical studies in MPS III: ERT, SRT and small molecules**

13:00 **Dr. Stephen Maricich, Allievex Corporation, Marblehead, Massachusetts, USA**
Study 250-202 of tralesinidase alfa in MPS IIIB

14:30 **Dr. Raj Mehra, Seelos Therapeutics, New York, USA**
Trehalose (SLS-005) a small molecule for MPS III, OPMD and SCA3

- **Session 3: Other MPS diseases**

15:00 **Dr. Mireille Tallandier, Inventiva Pharma, Daix, France**
iMProveS: a Phase IIa clinical trial of odiparcil for the treatment of adults with MPS VI

16:00 **Dr. Bettina Cockroft, Sangamo USA**
The ST-920 program for Fabry disease

16:15 **Experts discussion forum (1 hour) - Speakers only**

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➢ Session 4: Screening and diagnosis

9:30 **Dr. Zoltan Lukacs, Institute of Clinical Chemistry/Dept. of Pediatrics, University Medical Center Hamburg-Eppendorf, Germany**
Targeted screening for lysosomal storage diseases

10:00 **Prof. Lucy Raymond, Cambridge**
The next generation of children project: Whole genome sequencing for rapid diagnosis of severely Ill children in intensive care

10:30 **Dr. Francisco J. del Castillo, Genetic Services, Ramon y Cajal Hospital, Madrid, Spain**
Early detection of lysosomal diseases by screening of cases of idiopathic splenomegaly and/or thrombocytopenia with a next-generation sequencing gene panel **(Title TBC)**

11:00 **Dr. Ana Carolina Brusius-Facchin, Division of Medical Genetics, Hospital de Clinicas of Porto Alegre (HCPA), Brazil**
Sensitivity, advantages, limitations and clinical utility of targeted next-generation sequencing panels for the diagnosis of selected lysosomal storage disorders

➢ Session 5: Early stage and investigational treatments

11:30 **Dr. Maria Francisca Coutinho, Research and Development Unit, Human Genetics Dept., National Institute of Health, Porto, Portugal**
Targeting stable nucleic acid lipid particle (SNALP)-formulated siRNAs to the brain as a therapeutic approach in lysosomal storage disease-associated neuropathy

12:00 **Dr. Brian Bigger, Faculty of Medical and Human Sciences, University of Manchester, UK**
Innate immunity in MPS - or - Impact of the immune system on the safety and efficiency of ERT in LSDs **(Title TBC)**

12:30 **Dr. Anastasia Henry, Denali Therapeutics, South San Francisco, USA**
Intravenously-administered lysosomal enzyme using a blood-brain barrier transport vehicle

➢ Session 6: Round table Q & A

14:30 Session with scientific experts (1 hour) - Open to all registered participants
Chair: **Dr. Armand Bottani, Valais Hospital, Central Hospital Institute, Medical Genetics Services, Sion, Switzerland**