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