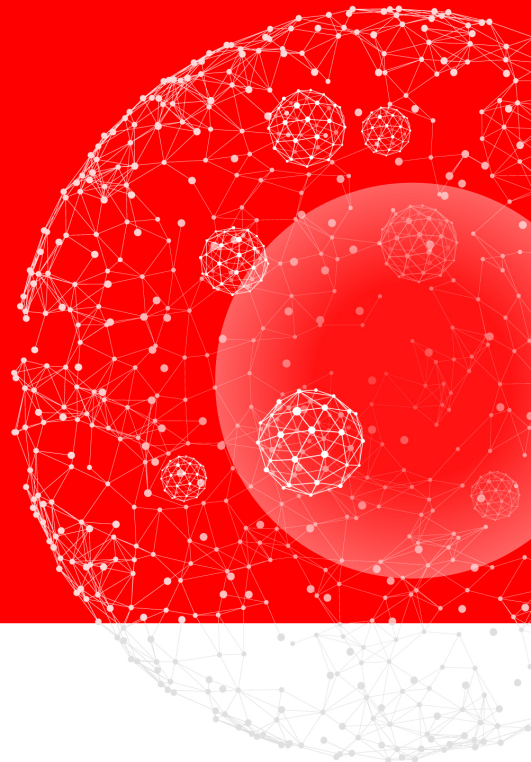




GSLSD2021

Global Symposium on Lysosomal Storage Disorders



Global Symposium on Lysosomal Storage Disorders

March 11–12, 2021

AGENDA

March 11, 2021

Time	Presentation Focus	Speaker
13:00	Welcome and introduction	Anthony Futerman (IL)
Session 1: Disorders of Lysosomes and Lysosome-related Organelles in the Pathology of LSDs		Chair: Anthony Futerman (IL)
13:05	Autophagy and its roles in cells and organisms	Eeva-Liisa Eskelinen (FI)
13:25	Lysosomal biogenesis and autophagy in disease	Chiara De Leonibus (IT)
13:45	Selective delivery of cargo substrates to lysosomes via autophagy and non-autophagic pathways	Carmine Settembre (IT)
14:05	Central nervous system inflammation and approaches to its inhibition in LSDs	Anthony Futerman (IL)
14:25	Mitochondrial dysfunction and neurodegeneration in LSDs	Michael R Duchen (UK)
14:45	Semaphorin 7A: A novel marker of disease activity in Gaucher disease	Nadia Belmatoug (FR)
15:05	Short Break	
15:20	Interactive, live Q&A and panel discussion: What clues have pathology provided for exploration of novel therapies?	Moderator: Anthony Futerman (IL) Discussants: All session 1 speakers
Session 2: Pathologic Cascades and Identification of Novel Treatment Targets (Part 1)		Chair: Derralynn Hughes (UK)
15:40	How to address the brain and systemic pathology of mucopolysaccharidoses	Joseph Muenzer (US)
16:00	New treatments for mucopolysaccharidoses: Lessons learned from disease pathways	Giancarlo Parenti (IT)
16:20	Gaucher disease in bone: From pathophysiology to practice	Derralynn Hughes (UK)
16:40	Closing remarks Day 1 and outline Day 2	Derralynn Hughes (UK)

March 12, 2021

Time	Presentation Focus	Speaker
13:00	Short review of Day 1 and outline for Day 2	Tim Cox (UK)
Session 2: Pathologic Cascades and Identification of Novel Treatment Targets (Part 2)		Chair: Derralynn Hughes (UK)
13:05	Progranulin replacement via gene therapy: Unexpected neurodegeneration	Defne Amado (US)
13:25	Approaches to correcting arylsulfatase A deficiency in MCL: Potential differences in biologic outcomes	Volkmar Gieselmann (DE)
13:45	Interactive, live Q&A and panel discussion: ERT, SRT, Chaperones; what targets and interventions are most promising?	Moderator: Derralynn Hughes (UK) Discussants: All session 2 speakers
Session 3: Pushing Toward Earlier Diagnosis and Individualized Treatment		Chair: Tim Cox (UK)
14:10	Laboratory diagnosis of lysosomal diseases: Newborn screening to treatment	Maria Fuller (AU)
14:30	Cell free DNA. Can it be used for <i>in utero</i> diagnosis of monogenic diseases like LSDs?	Rossa WK Chiu (HK)
14:50	Diagnosis of genetic diseases by rapid whole-genome sequencing and automated phenotyping and interpretation	Stephen Kingsmore (US)
15:10	Short Break	
15:25	Acid ceramidase as a disease-modifying target in sphingolipid diseases	Hans J.M.F.G. Aerts (NL)
15:45	Bridging the gap between vessels and nerves: Neurovascular dysfunction in Fabry disease	Ralf Baron (DE)
16:05	New approaches to monitoring progression in LSDs: Potential for improving patient management?	Kevin Mills (UK)
16:25	Interactive, live Q&A and panel discussion: Barriers to timely diagnosis, establishing a prognosis, and treatment initiation in patients with LSDs	Moderator: Tim Cox (UK) Discussants: All session 3 speakers
16:45	Personalized medicine for CLN7. What's next?	Timothy W Yu (US)
17:05	GSLSD 2021 wrap-up and meeting close	GSLSD 2021 SC Members