

Mitochondrial Medicine

30 November - 2 December 2020

Virtual Conference Agenda

Start (GMT)	Finish (GMT)	Presenter details
Monday, 30 November 2020		
13:00	13:10	Welcome <i>Scientific Programme Committee:</i> Marni Falk, The Children's Hospital of Philadelphia, USA Rita Horvath, University of Cambridge, UK Yasutoshi Koqa, Kurume University, Japan
13:10	13:55	Keynote 1 Introduction to the session <i>Chair: Marni Falk, The Children's Hospital of Philadelphia, USA</i>
13:10	13:40	Talk title TBC Mike Patane, Mitobridge, USA
13:40	13:55	Q&A <i>Chair: Marni Falk, The Children's Hospital of Philadelphia, USA</i> <i>Moderator: Treasa Creavin, Wellcome Genome Campus Scientific Conferences, UK</i>
13:55	14:20	Break
14:20	16:10	Session 1: Mitochondrial Medicine – Pharma and Funding Perspectives Introduction to the session <i>Chair: Rita Horvath, University of Cambridge, UK</i>
14:20	14:40	Clinical trials for mitochondrial diseases Petra Kaufmann, AveXis, USA
14:40	15:00	Talk title TBC Nigel Webb, RiboNova, USA
15:00	15:20	Reneo Pharmaceuticals: a case study in orphan drug development Alejandro Dorenbaum, Reneo Pharmaceuticals, USA
15:20	15:40	Poster lightning talks - pharma and funding perspectives
15:40	16:10	Q&A <i>Chair: Rita Horvath, University of Cambridge, UK</i> <i>Moderator: Marni Falk, The Children's Hospital of Philadelphia, USA</i>
16:10	16:30	Break

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16:30	18:20	Session 2: Pre-Clinical Mitochondrial Disease Models
		Introduction to the session <i>Chair: Yasutoshi Koga, Kurume University, Japan</i>
16:30	16:50	Targeting the cellular metabolism to treat tissue-specific mitochondrial diseases Rita Horvath, University of Cambridge, UK
16:50	17:10	Cardiolipin is required for membrane docking of mitochondrial ribosomes and protein synthesis Aleksandra Filipovska, University of Western Australia, Australia
17:10	17:30	Elucidating disease-relevant mitochondrial pathways by CRISPR-based functional genomics Martin Kampmann, University of California, San Francisco, USA
17:30	17:40	Pre-mRNA splicing control of mitochondria and energy metabolism <i>Alexis Jourdain, Broad Institute of MIT & Harvard, USA</i>
17:40	17:50	[11C]PK11195-PET brain imaging of the mitochondrial translocator protein in mitochondrial disease <i>Jelle van den Aamele, University of Cambridge, UK</i>
17:50	18:20	Q&A <i>Chair: Yasutoshi Koga, Kurume University, Japan</i> <i>Moderator: Rita Horvath, University of Cambridge, UK</i>
18:20	18:40	Break
18:40	19:00	Poster session I lightning talks
19:00	20:00	Poster session I

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13:00		15:10		Session 3: Clinical Trials
				Kindly sponsored by Reneo Pharmaceuticals
				Introduction to the session <i>Chair: Rita Horvath, University of Cambridge, UK</i>
				
13:00	13:20	A randomized, placebo-controlled study of sodium pyruvate in patients with mitochondrial disease Yasutoshi Koga, Kurume University, Japan		
13:20	13:40	Advancing primary mitochondrial disease clinical trials in the US Amel Karaa, Massachusetts General Hospital, USA		
13:40	14:00	Advancing primary mitochondrial disease clinical trials in the UK Grainne Gorman, Newcastle University, UK		
14:00	14:20	Poster lightning talks - clinical trials		
14:20	15:10	Q&A <i>Chair: Rita Horvath, University of Cambridge, UK</i> <i>Moderator: Yasutoshi Koga, Kurume University, Japan</i>		
15:10	15:30	Break		
15:30		17:00		Session 4: Regulatory Guidance for Therapeutic Development
				Introduction to the session <i>Chair: Marni Falk, The Children's Hospital of Philadelphia, USA</i>
15:30	15:50	Key scientific and regulatory aspects of therapeutic product development for primary mitochondrial diseases Patroula Smpokou, FDA, USA		
15:50	16:10	Therapeutic development for mitochondrial diseases: what have we learned from Japanese experience? Hidefumi Nakamura, National Center for Child Health and Development, Japan		
16:10	16:20	Collaborative model for diagnosis and treatment of very rare diseases: experience in Spain with thymidine kinase 2 deficiency <i>Carmen Paradas, Instituto de Biomedicina, Spain</i>		
16:20	16:30	Expanding and validating the biomarkers for mitochondrial diseases <i>Alessandra Maresca, IRCCS-ISNB, Italy</i>		
16:30	16:40	Developing the voice of mitochondrial disease patients through patient-populated registries <i>Philip Yeske, United Mitochondrial Disease Foundation, USA</i>		
16:40	17:10	Q&A <i>Chair: Marni Falk, The Children's Hospital of Philadelphia, USA</i> <i>Moderator: Rita Horvath, University of Cambridge, UK</i>		
17:10	17:30	Break		
17:30	17:50	Poster session II lightning talks		
17:50	18:50	Poster session II		

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Wednesday, 2 December 2020

13:00	15:10	Session 5: Informatics and Data Sharing to Support Precision Medicine
		Introduction to the session <i>Chair: Holger Prokisch, Technical University Munich, Germany</i>
13:00	13:20	Talk title TBC Ewan Birney, EMBL-EBI, UK
13:20	13:40	Genome-phenome data sharing and analysis with the RD-Connect GPAP: features for mitochondrial diseases Sergi Beltran, Centre Nacional Anàlisi Genòmica CNAG-CRG, Spain
13:40	14:00	Mitochondrial disease presenting early in life Lucy Raymond, University of Cambridge, UK
14:00	14:20	Talk title TBC Marni Falk, The Children's Hospital of Philadelphia, USA
14:20	14:30	Applications and development of massively parallel single-cell mitochondrial DNA sequencing in the context of multi-omic assays <i>Caleb Lareau, Stanford University, USA</i>
14:30	14:40	Mitochondrial diseases: a comprehensive overview of known disease-causing genes and systematic prioritisation of future candidates <i>Christoph Thiele, Helmholtz Zentrum München, Germany</i>
14:40	15:10	Q&A <i>Chair: Holger Prokisch, Technical University Munich, Germany</i> <i>Moderator: Marni Falk, The Children's Hospital of Philadelphia, USA</i>
15:10	15:30	Break
15:30	16:30	Session 6: Interactive Session
		Introduction to the session <i>Chair: Marni Falk, The Children's Hospital of Philadelphia, USA</i>
15:30	16:30	Interactive sessions - 30 mins each
16:30	16:45	Closing remarks
		Scientific Programme Committee: <i>Marni Falk, The Children's Hospital of Philadelphia, USA</i> <i>Rita Horvath, University of Cambridge, UK</i> <i>Yasutoshi Koga, Kurume University, Japan</i>