

Mitochondrial Medicine

30 November - 2 December 2020

Virtual Conference Agenda

Monday, 30 November 2020

13:00	13:10	Welcome <i>Scientific Programme Committee:</i> <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>
13:10	13:55	Keynote Introduction to the session <i>Chair: Marni Falk, The Children's Hospital of Philadelphia, USA</i>
13:10	13:40	<i>Mike Patane, Mitobridge, USA</i>
13:40	13:55	Q&A <i>Chair: Marni Falk, The Children's Hospital of Philadelphia, USA</i> <i>Moderator: Yasutoshi Koga, Kurume University, Japan</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 <i>Petra Kaufmann, AveXis, USA</i>
14:40	15:00	<i>Nigel Webb, RiboNova, USA</i>
15:00	15:20	<i>Niall O'Donnell, Reneo Pharmaceuticals, USA</i>
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

Mitochondrial Medicine

30 November - 2 December 2020

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	<i>Rita Horvath, University of Cambridge, UK</i>
16:50	17:10	Cardiolipin is required for membrane docking of mitochondrial ribosomes and protein synthesis <i>Aleksandra Filipovska, University of Western Australia, Australia</i>
17:10	17:30	Elucidating disease-relevant mitochondrial pathways by CRISPR-based functional genomics <i>Martin Kampmann, University of California, San Francisco, USA</i>
17:30	17:40	Short talk - selected from submitted abstracts
17:40	17:50	Short talk - selected from submitted abstracts
17:50	18:20	Q&A <i>Chair: Yasutoshi Koga, Kurume University, Japan</i> <i>Moderator: TBC</i>
18:20	18:40	Break
18:40	19:40	Poster session TBC

Mitochondrial Medicine

30 November - 2 December 2020

Tuesday, 1 December 2020

13:00 15:10 Session 3: Clinical trials		
Introduction to the session <i>Chair: Rita Horvath, University of Cambridge, UK</i>		
13:00	13:20	<i>Yasutoshi Koga, Kurume University, Japan</i>
13:20	13:40	Advancing primary mitochondrial disease clinical trials in the US <i>Amel Karaa, Harvard University, USA</i>
13:40	14:00	<i>Grainne Gorman, Newcastle University, UK</i>
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: TBC</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 & regulatory aspects of therapeutic product development for primary mitochondrial diseases <i>Patroula Smpokou, FDA, USA</i>
15:50	16:10	Therapeutic development for mitochondrial diseases: What have we learned from Japanese experience? <i>Hidefumi Nakamura, National Center for Child Health and Development, Japan</i>
16:10	16:20	Short talk - selected from submitted abstracts
16:20	16:30	Short talk - selected from submitted abstracts
16:30	17:00	Q&A <i>Chair: Marni Falk, The Children's Hospital of Philadelphia, USA</i> <i>Moderator: TBC</i>
17:00	17:20	Break
17:20	17:40	Poster lightning talks
17:40	18:40	Poster session

Mitochondrial Medicine

30 November - 2 December 2020

Wednesday, 2 December 2020

13:00	15:10	Session 5: Informatics and data sharing to support precision medicine
		Introduction to the session <i>Chair: TBC</i>
13:00	13:20	<i>Ewan Birney, EMBL-EBI, UK</i>
13:20	13:40	Genome-phenome data sharing and analysis with the RD-Connect GPAP: features for mitochondrial diseases <i>Sergi Beltran, Centre Nacional Anàlisi Genòmica CNAG-CRG, Spain</i>
13:40	14:00	Mitochondrial disease presenting early in life <i>Lucy Raymond, University of Cambridge, UK</i>
14:00	14:20	<i>Marni Falk, The Children's Hospital of Philadelphia, USA</i>
14:20	14:30	Short talk - selected from submitted abstracts
14:30	14:40	Short talk - selected from submitted abstracts
14:40	15:10	Q&A <i>Chair: TBC</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
15:30	16:30	Interactive sessions
16:30	16:45	Closing remarks
		<i>Scientific Programme Committee:</i> <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>