Wednesday 11 December 2019

12:00-13:00  **Registration** with buffet lunch

13:00-13:20  **Welcome and Conference Overview**  
**Patrick Chinnery**  
*University of Cambridge, UK*

13:20-15:00  **Session 1 Mitochondrial Medicine: Current Landscape of Therapeutic Opportunities**  
*Chair: Patrick Chinnery, University of Cambridge, UK*

13:20  Regulatory Environment for Novel Therapeutic Development in Mitochondrial Disease  
**Michio Hirano**  
*University of Columbia, USA*

13:45  Developing new treatments in partnership: what does industry need from academics, and what do academics need from industry?  
**Paul Thompson**  
*Mission Therapeutics, UK*

14:10  Pharma presentations

14:40-15:00  **Round Table Panel Discussion**

15:00-16:00  **Afternoon Tea**  
Networking between academics, clinicians and industry

16:00-17:20  **Session 2: pre-clinical mitochondrial disease models I: Academic-Pharma Spin-out and Partnership Models**  
*Chair: Vamsi Mootha, Harvard Medical School, USA*

16:00  Pre-clinical modeling across evolutionarily-distinct animals and patient cells to optimize mitochondrial disease precision therapies  
**Marni Falk**  
*The Children's Hospital of Philadelphia, USA*

16:25  Reproducing mitochondrial hereditary optic neuropathy in neurons: light on pathogenic mechanisms to fight back blindness  
**Valeria Tiranti**  
*IRCCS Foundation Neurological Institute "C.Besta", Italy*
Dynamics of heteroplasmy in neural differentiation at single cell resolution
Michele Frison
University of Cambridge, UK

Mitochondria-targeting hydrogen sulfide extends life- and health-span and healthspan in C. elegans: implications for primary mitochondrial disease
Mike Cooke
University of Exeter, UK

17:20-18:00 Lightning Talks
Chair: Vamsi Mootha, Harvard Medical School, USA

18:00-19:30 Drinks Reception with Poster Session
19:30 prompt Buffet Dinner
19:30 Cash Bar

Thursday 12 December 2019

09:00-10:05 Session 3: pre-clinical mitochondrial disease models II-
Chair: Marni Falk, University of Pennsylvania, USA

09:00 Pre-clinical mitochondrial disease vertebrate models advance therapeutic development from academia to pharma
Sherine Chan
Medical University of South Carolina, USA

09:25 Mouse models of mitochondrial diseases
Jim Stewart
MPI Biology of Ageing, Germany

09:50 Mitochondrial DNA depletion syndrome caused by mutation in ANT1: A de novo dominant causing germline mosaicism with dysregulated mitophagy
Joanna Poulton
University of Oxford, UK

10:05-11:05 Morning Coffee

11:05-12:50 Session 4 Translatable biomarkers of disease progression
Chair: Marni Falk, University of Pennsylvania, USA

11:05 Mitochondrial biomarker GDF15: usefulness and development of diagnostic device from laboratory inspection to automated inspection
Yasutoshi Koga
Kurume University School of Medicine, Japan
11:25 Mitochondrial disease pathophysiology reveals tools for diagnosis and treatment
   Anu Suomalainen
   University of Helsinki, Finland

11:50 Human fibroblasts and Omic technologies as models for functional validation beyond the clinical exome
   David Thorburn
   Murdoch Children’s Res. Institute, Australia

12:05-12:30 Round Table Panel Discussion

12:30-14:00 Lunch

14:00-15:30 Session 5: Seeking Impact: Outcome measure selection for translational and clinical research -- International perspective
   Chair: Vamsi Mootha, Harvard Medical School, USA

14:00 The present state of the translational research for mitochondrial diseases in Japan: A sprout of many hopeful drugs.
   Akira Ohtake
   Saitama Medical University, Japan

14:20 UK Perspective - Seeking Impact: Outcome measure selection for translational and clinical research
   Shamima Rahman
   University College of London, UK

14:40 US Perspectives - Seeking Impact: Outcome measure selection for translational and clinical research
   Amy Goldstein
   Children’s Hospital of Philadelphia, USA

15:00 Quantitative Assessment of Mitochondrial Myopathy
   Zarazuela ZolkpliCunningham
   Children’s Hospital of Philadelphia, USA

15:15 Chemical inhibition of mtDNA transcription for treatment of cancer
   Nina Bonekamp
   Max Planck Institute for Biology of Ageing

15:30-16:00 Round Table Panel Discussion

16:00-16:30 Afternoon Tea

16:30-17:10 Lightning Talks
   Chair: Vamsi Mootha, Harvard Medical School, USA

17:10-18:30 Drinks Reception with Poster Session

18:30 Coach to Kings College

Silver Service Conference Dinner
Friday 13 December 2019

09:00-10:00  **Keynote Speaker**  
*Chair: Vamsi Mootha, Harvard Medical School, USA*

Blue skies research: academic discoveries, commercial outputs and patient benefits  
*Stephen Jackson*  
*The Gurdon Institute, University of Cambridge, UK*

10:00-10:30  **Morning Coffee**

10:30-12:30  **Session 6 Non-Drug Therapies**  
*Chair: Patrick Chinnery, University of Cambridge, UK*

10:30  Mitochondrial Transfer and Cell Fate Transitions  
*Micahel Teitell*  
*University of California Jonsson Comprehensive Cancer Center, USA*

11:00  Mitochondrial Augmentation Therapy: Can isolated mitochondria re-enter cells? The path from an idea to a possible solution for mitochondrial diseases  
*Natalie Yivgi Ohaha*  
*Minovia Therapeutics, Israel*

11:30  Probing Mitochondrial Chemical Biology with Molecular Probes  
*Shana O. Kelly*  
*University of Toronto, Canada*

12:00  Yeast, worm, patient’s fibroblasts and zebrafish as models for drug repositioning of molecules targeting POLG-related diseases  
*Enrico Baruffini*  
*University of Parma, Italy*

12:15  An In Vitro Proof-of-Principle Study to Test the Efficacy of Translational Read-Through Therapy for Mitochondrial Disorders  
*Joyeeta Rahman*  
*UCL Institute of Child Health, UK*

12:30-13:00  **Round Table Panel Discussion**

13:00 - 14:15  **Lunch**
Session 7 Advocating for mitochondrial medicine therapeutic development: International perspective, support, and collaborative potential

Chair: Patrick Chinnery, University of Cambridge, UK, Marni Falk, University of Pennsylvania, USA, Vamsi Mootha, Harvard Medical School, USA

14:15 Phil Yeske
UMDF, USA

14:25 Sean Murray
The Mitochondrial Foundation, Australia

14:35 Piero Santantonio
MitoCon, Italy

14:45 Blaine Penny
MitoCanada, Canada

14:55 Alison Maguire
Lily Foundation, UK

15:05-15:40 Round Table Panel Discussion

15:40-15:50 Closing remarks
Patrick Chinnery
University of Cambridge, UK

16:05 Coaches depart to Stansted Airport via Heathrow Airport

16:15 Coaches depart to Cambridge City Centre and Train Station,