



UMDF Mitochondrial Medicine 2024 Scientific/Clinical Program

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<https://umdfconference.org/scientific-program/>

Wednesday, June 26

Speaker & Topic

Wed Morning - Platform Session 1 - Advances in Disease Models for Investigating Mitochondrial Diseases – Superior Room D

8:00a-8:30a	<p>Welcome and Opening Remarks <i>Brian Harman, UMDF President & CEO and Margaret Moore, UMDF Associate Director of Support & Education</i> Patient Story – <i>Jared Goettemoeller, TK2d Patient</i></p>
8:30a-9:00a	<p>Mitochondrial Double-Stranded RNA as a New DAMP to Activate the Immune Response in Mitochondrial Disease <i>Carla Koehler, PhD</i> <i>University of California Los Angeles</i></p>
9:00a-9:30a	<p>Brain Organoids for Leigh Syndrome Drug Discovery <i>Alessandro Prigione, MD, PhD</i> <i>Heinrich Heine University, Dusseldorf Germany</i></p>
9:30a-10:00a	<p>Development of Cortical Organoids to Model m.3243A>G Disease and Understand Cell Specificity <i>Denisa Hathazi, PhD</i> <i>University of Cambridge, UK</i></p>
<i>10:00a-10:30a</i>	<i>Break</i>
10:30a-11:00a	<p>The Distribution and Functional Impact of MELAS Mutation m.3243A>G in Cardiac Cells, and Novel Approaches to Restore Cellular Dysfunction <i>Alexander Kreymerman, PhD</i> <i>Harvard University, Department of Stem Cell and Regulative Biology Cambridge, MA</i></p>
11:00a-11:30a	<p>Invited Abstract Short Talks Induced Pluripotent Stem Cell Based Cardiac Model of MELAS Disease <i>Graeme Preston, PhD, Mayo Clinic, Rochester, MN</i> Modeling Diabetes in MELAS Disease Using Induced Pluripotent Stem Cell Based Paretatic Organoids <i>Quinn Peterson, PhD – Mayo Clinic, MN</i></p>
11:30a-12:00p	<p>Abstract Presentations – Non CME <i>Shilpa Iyer, PhD</i> <i>Lia Mayorga, MD, PhD</i></p>
12:00p-12:30p	<p>Abstract Presentations – Non CME <i>Chen Ding, PhD</i> <i>Madhurima Saha, PhD</i></p>
<i>12:30p-2:00p</i>	<i>Lunch – Hope Ballroom DE</i>

Wednesday, June 26

Wed Afternoon - Platform Session 2 - Mitochondria and Metabolic Plasticity - Superior Room D

2:00p-2:30p	Metabolic Causes of Mitochondrial Myopathy and Tools for Treatment <i>Anu Suomalainen-Wartiovaara, MD, PhD</i> <i>University of Helsinki, Finland</i>
2:30p-3:00p	Understanding the Role of Oxidative Metabolism in Physiology and Cancer <i>Kivanc Birsoy, PhD</i> <i>The Rockefeller University, New York, NY</i>
3:00p-3:30p	Reconfiguring Mitochondrial Metabolic Networks Support Cell State Transitions <i>Lydia Finley, PhD</i> <i>Memorial Sloan Kettering Cancer Center, New York, NY</i>
3:30p-4:00p	<i>Break</i>
4:00p-4:30p	Mitochondrial Metabolism in Innate Immune Cell Response <i>Jing Fan, PhD</i> <i>Morgridge Institute for Research/University of Wisconsin-Madison</i> <i>Madison, WI</i>
4:30p-5:00p	Abstract Presentations – Non CME <i>Ren Zhang, PhD</i> <i>Riley Wedan, BS</i>
5:00p-5:30p	Abstract Presentations – Non CME <i>Shahreen Amin, MD, PhD</i> <i>Jose Domingo Barrera-Paez</i>
5:30p-7:30p	<i>Break and Poster Sessions – Center Street Room A</i> <i>Even Numbers Stay at Posters - NON CME</i>

Thursday, June 27

Speaker & Topic

Thu Morning - Platform Session 3 - Disorders of Mitochondrial DNA Integrity - Superior Room D

8:00a-8:30a	mtDNA Rearrangements: Mechanisms of Formation and Approaches to Therapy <i>Carlos Moraes, PhD</i> <i>University of Miami, Miami, FL</i>
8:30a-9:00a	Reprogramming Metabolism by Inhibition of mtDNA Gene Expression <i>Nils-Goran Larsson, PhD</i> <i>Karolinska Institutet, Stockholm, Sweden</i>
9:00a-9:30a	Expanding the Natural History of Pediatric Single Large Scale mtDNA Deletion Syndromes <i>Rebecca Ganetzky, MD</i> <i>Children's Hospital of Philadelphia, Philadelphia, PA</i>

Thursday, June 27

Speaker & Topic

Thu Morning - Platform Session 3 - Disorders of Mitochondrial DNA Integrity - Superior Room D

9:30a-10:00a	CPEO Spectrum Disorder - a Redefinition of Old Terms <i>Mark Tarnopolsky, PhD, MD, FRCP(C)</i> <i>McMaster University, Health Sciences Centre, Toronto, Canada</i>
10:00a-10:30a	<i>Break</i>
10:30a-11:00a	Describing Caregiver Burden in Families Affected by Pediatric Single Large-scale mtDNA Deletion Syndromes (SLSMDS) <i>Elizabeth Reynolds, PhD</i> <i>The Champ Foundation, Durham, NC</i>
11:00a-11:30a	Shifting Heteroplasmy with PBGENE-PMM: Gene Editing Therapy for m.3243A>G Associated Mitochondrial Myopathy <i>Wendy Shoop, PhD</i> <i>Precision Biosciences, Durham, NC</i>
11:30a-12:00p	Molecular Consequences of POLG Mutations and New Avenues of Treatment <i>Maria Falkenberg, PhD University of Gothenburg, Göteborg, Sweden</i>
12:00p-12:30p	Abstract Presentations – Non CME <i>Caterina Garone, MD, PhD</i> <i>Kenneth Myers, MD, PhD, FRCPC</i>
12:30p-2:00p	<i>Lunch – Hope Ballroom DE</i>

Thu Afternoon - Platform Session 4 - Development of Improved Outcome Measures for Mitochondrial Clinical Trials - Superior Room D

2:00p-2:30p	Transforming Mitochondrial Disease Diagnostics through the 100,000 Genomes Programme <i>Shamima Rahman, FRCP, FRCPCH, PhD</i> <i>University College of London, United Kingdom</i>
2:30p-3:00p	Development of a Novel Outcome Measure for Rare Pediatric Neuromuscular Disease <i>Linda Lowes PT, PhD</i> <i>Nationwide Children's Hospital, Columbus, OH</i>
3:00p-3:30p	Validation of Digital Wearables in Mitochondrial Disease <i>Zuela Zolkipli-Cunningham, MD</i> <i>Children's Hospital of Philadelphia, Philadelphia, PA</i>
3:30p-4:00p	<i>Break</i>
4:00p-4:30p	Improving the Assessment of Walking Function in Controlled & Natural Environments with AI-Enabled Insoles <i>Damiano Zanotto, PhD, Stevens Institute of Technology, Hoboken, NJ</i>

Thursday, June 27		Speaker & Topic
Thu Afternoon - Platform Session 4 - Development of Improved Outcome Measures for Mitochondrial Clinical Trials		
4:30p-5:00p	Abstract Presentations – Non CME <i>Jean Flickinger, PT, DPT, PCS</i> <i>Sandra R. Bacman, PhD</i>	
5:00p-5:30p	Abstract Presentations – Non CME <i>Chad Glasser, PharmD</i> <i>Andrea Cortes Fernandez, MD</i>	
5:30-6:00p	Abstract Presentations – Non CME <i>Cristy Balcells, RN, MSN</i> <i>Elizabeth McCormick, MS, LCGC</i>	
6:00p-8:00p	Poster Sessions - Odd Numbers Stay at Posters - Reception Note: Poster Sessions are NON CME – Center Street Room A	
Friday, June 28		Speaker & Topic
Fri Morning - Platform Session 5 - Combined Sessions/Clinical Trial Updates - NON CME		
8:00a-10:00a	<p align="center">Combined Sessions for All Attendees - NON CME</p> <p align="center">Welcome: UMDF and the State of the Mitochondrial Medicine Community and IMPACT</p> <p align="center">Join us for the accelerator Big Pitch Presentations! Hope Ballroom</p>	
10:00a-10:30a	<i>Break</i>	
10:30a-12:30p	<p align="center"><u>Clinical Trial Updates - NON CME:</u></p> <p align="center">Presenter Line-Up is Available in the App Superior Room D</p>	
12:30p-2:00p	<p align="center"><i>Lunch - Networking by Geographic (for those interested in connecting with attendees in their regions)</i></p> <p align="center">Hope Ballroom</p>	
Fri Afternoon - Platform Session 6 - Mitochondrial Dysfunction and its Impact on Mental Health		
2:00p-2:30p	<p align="center">Psychiatric Presentation in Mitochondrial Disease <i>Amy Goldstein, MD</i> <i>Children's Hospital of Philadelphia, Philadelphia, PA</i></p>	

Friday, June 28		Speaker & Topic
Fri Afternoon - Platform Session 6 - Mitochondrial Dysfunction and its Impact on Mental Health - Superior Room D		
2:30p-3:00p	<p>The Role of Mitochondrial Function in Mental Illness <i>Ana Cristina Andreatza, Pharm, PhD</i> <i>University of Toronto, Toronto, Canada</i></p>	
3:00p-3:30p	<p>Mitochondrial Energetics May Elevate Risk of Treatment-Emergent Mania <i>Mark A. Frye, MD</i> <i>Mayo Clinic College of Medicine, Rochester, MN</i></p>	
3:30p-4:00p	<i>Break</i>	
4:00p-4:30p	<p>Role of Mitochondria in the Neurobiological Mechanisms of Bipolar Disorder <i>Tadafumi Kato, MD, PhD (pre-recorded)</i> <i>Juntendo University Graduate School of Medicine, Tokyo, Japan</i></p>	
4:30p-5:00p	<i>Mental Health - Panel Discussion with all Speakers</i>	
5:00p-5:30p	<p>Abstract Cash Award Presentations <i>Philip Yeske, PhD, UMDF Science & Alliance Officer</i></p>	
5:30p-9:30p	<p>UMDF Evening of Energy Reception and Banquet Celebration for ALL Attendees Hope Ballroom</p>	
Saturday, June 29		Speaker & Topic
Sat Morning - Platform Session 7 – Therapeutic Development for Mitochondrial Disease: Lessons learned from other Rare Diseases - Superior Room D <i>Moderators: Zuela Zolkipli Cunningham, MD</i>		
8:00a-8:15a	<p>Therapeutic Landscape for Mitochondrial Disease <i>Philip Yeske, PhD, UMDF Science & Alliance Officer</i></p>	
8:15a-9:00a	<p>Recounting the Adventure of SMA from a Deadly to Manageable Disease <i>Richard S. Finkel, MD</i> <i>St. Jude Children’s Research Hospital, Memphis, TN</i></p>	
9:00a-9:30a	<p>Efforts Toward Approved Therapies for Barth Syndrome <i>Hilary Vernon, MD, PhD</i> <i>Johns Hopkins University, Baltimore, MD</i></p>	
9:30a-10:00a	<p>The Drug Approval Journey in Friedreich’s Ataxia and New Insights into Post-Approval Challenges <i>David R. Lynch, MD, PhD</i> <i>Children’s Hospital of Philadelphia, Philadelphia, PA</i></p>	

Saturday, June 29

Speaker & Topic

**Sat Morning - Platform Session 7 – Therapeutic Development for Mitochondrial Disease:
Lessons learned from other Rare Diseases - Superior Room D
Moderators: Zuela Zolkipli Cunningham, MD**

<i>10:00a-10:30a</i>	<i>Break</i>
10:30a-11:00a	Lessons Learned from the Drug Approval Journey in Metabolic Disorder David Whiteman, BM BCh FAAP FACMG Rare Disease Physician, Advocate and Principal Consultant, Spurwink Discovery, Cape Elizabeth, ME
11:00a-12:00p	Therapeutic Development Panel Discussion All Speakers Listed Above Lindsay Marjoram, Director of Research, Barth Syndrome Foundation Ruth Acton, Board Treasurer, Friedreich’s Ataxia Research Alliance Angela Paradis, ScD, Senior Medical Director, Neuromuscular Diseases at Biogen
12:00p-12:30p	Closing
<i>12:30p-2:00p</i>	<i>Lunch - Stay and Network or Grab n Go Hope Ballroom</i>