

United Mitochondrial Disease Foundation Scientific Program Agenda

Mitochondrial Medicine 2018: Nashville Mitochondrial Chemical Biology

June 27-30, 2018 – Scientific Sessions
June 28-30 – Patient/Family and LHON Sessions
Sheraton Music City, Nashville, TN

Day 1: June 27 (Wednesday)

Morning Platform Session (1) – Platform Title: *Advances in Mitochondrial Biochemistry and Metabolism*

- 8:00 a.m. Welcome – Vamsi Mootha, MD, Course Chair
- 8:15 a.m. Topic Title: Mitochondria, Metabolism and Cellular Coordination
Speaker Name: *Jared Rutter, PhD, University of Utah, Salt Lake City, UT*
- 8:45 a.m. Topic Title: Defining Mitochondrial Protein Function through Systems Biochemistry
Speaker Name: *David J. Pagliarini, PhD, Morgridge Institute for Research at UW-Madison, WI*
- 9:15 a.m. Abstract Presentations (2)
- 9:45 a.m. Break**
- 10:15 a.m. Topic Title: Bioenergetic Rescue of Human Mitochondrial Mutations
Speaker Name: *Pere Puigserver, PhD, Dana-Farber Cancer Institute, Boston, MA*
- 10:45 a.m. Abstract Presentations (6)
- 12:15 p.m. NAMDC Update – Michio Hirano, MD
- 12:30 p.m. Lunch**

Afternoon Platform Session (2) – Platform Title: *New Chemical Tools for Investigating Mitochondria*

- 2:00 p.m. Topic Title: Chemo-Genetic Tools for Probing Mitochondrial Proteomes and Transcriptomes
Speaker Name: *Alice Ting, PhD, Stanford University, Stanford, CA*
- 2:30 p.m. Topic Title: Activity-Based Sensing of Metal and Redox Biology
Speaker Name: *Christopher J. Chang, PhD, UC Berkeley, CA*
- 3:00 p.m. Abstract Presentations (2)
- 3:30 p.m. Break**
- 4:00 p.m. Topic Title: Targeting Mitochondria
Speaker Name: *Shana Kelley, PhD, University of Toronto, Canada*
- 4:30 p.m. Abstract Presentations (4)
- 5:30 p.m. Break & Poster Reception and Cash Bar (non cme) - Even Numbers Stay at Posters**
- 7:30 p.m. Adjourn**

Day 2: June 28 (Thursday)

Morning Platform Session (3) – Platform Title: *Mitochondrial Metabolomics: from Basic Mechanisms to Biomarkers*

- 8:00 a.m. Topic Title: Plasma Metabolic Profiles of Mitochondrial Disease
Speaker Name: *Rohit Sharma, MD, PhD, Massachusetts General Hospital, Boston, MA*
- 8:30 a.m. Topic Title: Broad Scale Untargeted Metabolomics Provides Functional Evidence for Diagnosis of Inborn Errors of Metabolism
Speaker Name: *Sarah Elsea, PhD, Baylor College of Medicine, Houston, TX*
- 9:00 a.m. Topic Title: Imaging Mass Spectrometry
Speaker Name: *Richard Caprioli, PhD, Vanderbilt University, Nashville, TN*
- 9:30 a.m. Abstract Presentations (2)
- 10:00 a.m. Break**

10:30 a.m. Topic Title: Integration of High-Resolution Metabolomics, Redox Proteomics, Transcriptomics and Respiratory Activities to Understand Complexities of Mitochondria-Cell Signaling
 Speaker Name: *Dean P. Jones, PhD, Emory University, Atlanta, GA*

Clinical Platform Session Track (organized by Mitochondrial Medicine Society)
Management of Fatigue and Exercise Intolerance in Mitochondrial Disease

- 11:00am MMS Business Updates
- 11:15am-11:45am Mitochondrial Care Network - Parikh
- 11:50am-12:30pm Risks and Benefits of Exercise in Mitochondrial Disease Patients
 Speaker: *Mark Tarnopolsky, MD, PhD*
- 12:30pm to 1pm – Lunch Served
- 1:00pm-1:40pm Exercise Testing for Mitochondrial Disease Patients
 Speaker: *Tania Taivassalo, PhD*
- 1:40pm-2:20pm – Fatigue Characteristic and Fatigue Outcome Measures in Mitochondrial Disease
 Speaker: *Grainne Gorman, MD*
- 2:20pm-2:30pm Closing Remarks Goldstein

11:00 a.m. Abstract Presentations (6)

12:30 p.m. Lunch

Afternoon Platform Session (4) – Platform Title: Drug discovery: from Structure Based Approaches to High Throughput Screening

2:30 p.m. Topic Title: Mitochondrial Drug Development: a Multi-Partner Endeavor
 Speaker Name: *Jan Smeitink, MD, PhD, Radboud Center for Mitochondrial Medicine, Radboudumc, Nijmegen, The Netherlands*

3:00 p.m. Topic Title: Small Molecule Inhibitors in Mitochondrial Protein Import
 Speaker Name: *Carla Koehler, PhD, University of California, Los Angeles, CA*

3:30 p.m. Break

4:00 p.m. Topic Title: Model Organisms for N=1 Drug Discovery
 Speaker Name: *Ethan Perlstein, PhD, CEO, Perlara, South San Francisco, CA*

4:30 p.m. Topic Title: Therapeutic Cross-training: High-throughput Screening Across Evolutionary-Distinct Genetic Models to Optimize Precision Mitochondrial Disease Therapies
 Speaker Name: *Marni Falk, MD, Children's Hospital of Philadelphia, PA*

5:00 p.m. Abstract Presentations (3)

5:45 p.m. Break & Posters Reception Odd Numbers Stay at Posters

Day 3: June 29 (Friday)

TENTATIVE Morning Platform Session (5) – Platform Title: Clinical Trials Updates (combined session with scientific, clinician, LHON attendees, patients and families)

8:00 a.m. UMDF Welcome – Brent Fields, UMDF Board Chair and Mitochondrial Disease Community Registry - Update for All

8:15 a.m. -10:30 a.m. History of Clinical Trials in Mitochondrial Disease
 Speaker: *Bruce H. Cohen, MD*

Clinical Trial Updates Session 1: LHON Specific
 Speakers to be determined

10:30 a.m. Break

11:00 a.m. to 12:30 p.m. Clinical Trial Updates Session 2: Primary Mitochondrial Diseases
 Speakers to be determined

Planning an Externally-led Patient-Focused Drug Development Meeting
 Speaker: *Philip Yeske, PhD*

12:30 p.m. Lunch

Afternoon Platform Session (6) – Targeting Mitochondria in Common Diseases

2:00 p.m.	Topic Title: Small Molecule Activators of Aldehyde Dehydrogenases Speaker Name: <i>Daria Mochly-Rosen, PhD, Stanford University, Stanford, CA</i>
2:30 p.m.	Topic Title: Medicines for Malaria and African Sleeping Sickness Speaker Name: <i>Kiyoshi Kita, PhD, Nagasaki University, Japan</i>
3:00 p.m.	Topic Title: Neuroprotective Effects of Inhibition of the Mitochondrial Pyruvate Carrier Speaker Name: <i>Anne N. Murphy, PhD, University of California San Diego, La Jolla, CA</i>
3:30 p.m.	Break
3:45 p.m.	Topic Title: Mitochondria and Diabetes Speaker Name: <i>Gerald I. Shulman, MD, PhD, Yale University School of Medicine, New Haven, CT</i>
4:15 p.m.	Topic Title: UMDF Funded Projects Speaker Name: <i>Philip Yeske, PhD, UMDF Science & Alliance Officer, Pittsburgh, PA</i>
4:30 p.m.	Adjourn
6:00 p.m.	Reception
7:00 p.m.	Friday Night Banquet and Awards Ceremony

A 5-minute question and answer period is included in each of the speaker's allotted time.

Day 4: June 30 (Saturday)

Morning Platform Session (7) – Platform Title: Mitochondrial Stress Responses

8:00 a.m.	MSeqDR Workshop Moderator: Marni Falk, MD and Colleen Clarke Muraresku, CGC
9:00 a.m.	Topic Title: TBD Speaker Name: <i>TBD</i>
9:30 a.m.	Topic Title: Compensatory Responses to Mitochondrial Toxicity Speaker Name: <i>Kendall B. Wallace, PhD, University of Minnesota Medical School, Duluth, MN</i>
10:00 a.m.	Break
10:30 a.m.	Topic Title: Manipulating Mitochondrial ROS and Oxidative Damage as Therapeutic Strategies Speaker Name: <i>Mike Murphy, PhD, MRC Mitochondrial Biology Unit, University of Cambridge</i>
11:00 a.m.	Topic Title: Stress Responses in Mitochondrial Disease Speaker Name: <i>Anu Suomalainen, MD, PhD, University of Helsinki, Finland</i>
11:30 a.m.	Closing – Vamsi Mootha, MD