Day 1: Wednesday, June 27, 2018

**Morning Platform Session (1)  Hermitage ABC**

*Advances in Mitochondrial Biochemistry and Metabolism*

8:00 am  Welcome  
Vamsi K. Mootha, MD, Course Chair, Harvard Medical School, Boston, MA

8:15 am  Mitochondria, Metabolism and Cellular Coordination  
Jared Rutter, PhD, University of Utah, Salt Lake City, UT

8:45 am  Defining Mitochondrial Protein Function through Systems Biochemistry  
David J. Pagliarini, PhD, Morgridge Institute for Research at UW-Madison, WI

9:15 am  Abstract Presentations (2)

9:45 am  Break

10:15 am  Bioenergetic Rescue of Human Mitochondrial Mutations  
Pere Puigserver, PhD, Dana-Farber Cancer Institute, Boston, MA

10:45 am  Abstract Presentations (6)

12:15 pm  North American Mitochondrial Disease Consortium (NAMDC) Update  
Michio Hirano, MD, Columbia University, New York, NY

12:30 pm  Lunch

**Afternoon Platform Session (2)  Hermitage ABC**

*New Chemical Tools for Investigating Mitochondria*

2:00 pm  Chemo-Genetic Tools for Probing Mitochondrial Proteomes and Transcriptome  
Alice Ting, PhD, Stanford University, Stanford, CA

2:30 pm  Activity-Based Sensing Approaches to Studying Metal and Redox Biology  
Christopher J. Chang, PhD, University of California, Berkeley, CA

**NOTE:** Abstract times follow this schedule and a five minute question and answer period is included in each of the speaker’s allotted time.
3:00 pm  Abstract Presentations (2)

3:30 pm  Break

4:00 pm  Integration of High-Resolution Metabolomics, Redox Proteomics, Transcriptomics and Respiratory Activities to Understand Complexities of Mitochondria-Cell Signaling
Dean P. Jones, PhD, Emory University, Atlanta, GA

4:30 pm  Abstract Presentations (4)

5:30 pm  Break & Poster Reception and Cash Bar (non cme)
Even Numbers Stay at Posters

7:30 pm  Adjourn

Day 2: Thursday, June 28, 2018

Morning Platform Session (3)  Hermitage ABC
Mitochondrial Metabolomics: from Basic Mechanisms to Biomarkers

8:00 am  Plasma Metabolic Profiles of Mitochondrial Disease
Rohit Sharma, MD, PhD, Massachusetts General Hospital, Boston, MA

8:30 am  Broad Scale Untargeted Metabolomics Provides Functional Evidence for Diagnosis of Inborn Errors of Metabolism
Sarah Elsea, PhD, Baylor College of Medicine, Houston, TX

9:00 am  Imaging Mass Spectrometry
Richard Caprioli, PhD, Vanderbilt University, Nashville, TN

9:30 am  Abstract Presentations (2)

10:00 am  Break

10:30 am  Targeting Mitochondria
Shana Kelley, PhD, University of Toronto, Canada
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<th>Event</th>
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<tr>
<td>11:00 am</td>
<td><strong>Abstract Presentations (6)</strong></td>
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<td>12:30 pm</td>
<td>Lunch</td>
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<td><strong>Afternoon Platform Session (4)</strong></td>
<td><strong>Hermitage ABC</strong></td>
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<tr>
<td>2:30 pm</td>
<td><strong>Mitochondrial Drug Development: a Multi-Partner Endeavor</strong></td>
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<td>Jan Smeitink, MD, PhD, Radboud Center for Mitochondrial Medicine, Radboudumc, Nijmegen, The Netherlands</td>
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<td>3:00 pm</td>
<td><strong>Small Molecule Inhibitors in Mitochondrial Protein Import</strong></td>
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<td>Carla Koehler, PhD, University of California, Los Angeles, CA</td>
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<td>3:30 pm</td>
<td>Break</td>
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<tr>
<td>4:00 pm</td>
<td><strong>Model Organisms for N=1 Drug Discovery</strong></td>
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<td>Ethan Perlstein, PhD, CEO, Perlara, South San Francisco, CA</td>
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<td>4:30 pm</td>
<td><strong>Therapeutic Cross-training: High-throughput Screening Across</strong></td>
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<td><strong>Evolutionary-Distinct Genetic Models to Optimize Precision Mitochondrial Disease Therapies</strong></td>
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<td>Marni Falk, MD, Children’s Hospital of Philadelphia, PA</td>
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<td>5:00 pm</td>
<td><strong>Abstract Presentations (3)</strong></td>
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<td>5:45 pm</td>
<td>Break &amp; Posters Reception Odd Numbers Stay at Posters</td>
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Day 3: Friday, June 29, 2018

Morning Platform Session (5)  
Clinical Trials Updates and Challenges  
Moving Drugs from Concept to Clinic

8:00am  Welcome and Clinical Trials Session  
(Combined with Scientific, Clinician, Patient, Family and LHON Attendees)  
Brent Fields, Chuck Mohan, Philip Yeske, PhD

8:10am  History of Clinical Trials in Mitochondrial Disease  
Bruce H. Cohen, MD, Akron Children’s Hospital, Akron, OH

8:40am  Clinical Trial Updates Session 1: Small Molecule Approaches  
- Mitochondria Disease Program Update  
  Reenie McCarthy, Chief Executive Officer, Stealth BioTherapeutics  
- Safety, Efficacy, and Pharmacodynamics of Omaveloxolone in Mitochondrial Myopathy Patients (MOTOR Trial): Part 1 Results  
  Karen Lindhardt Madsen, PhD, University of Copenhagen  
- The KHERGY study: an exploratory, double-blind, randomized, placebo-controlled, two-way cross-over phase II trial in m.3243A>G patients  
  Jan Smeitink, CEO, Khondrion

9:45am  Break

10:15am  Clinical Trial Updates Session 2: Vision Disorders  
Moderators: Lissa Poincenot and Nancy Newman, MD  
- Overview of Mitochondrial Vision Disorders  
  Nancy Newman, MD  
- GenSight: What we have Done, What we are Doing  
  Barrett Katz, MD, MBA, Chief Medical Officer, GenSight Biologics  
- Santhera’s Real World Experience with Idebenone in LHON. Update on Programs  
  Xavier Lloria, Medical Affairs Director, Santhera Pharmaceuticals  
- LHON Development Update  
  Jim Carr, PharmD, Chief Clinical Development Officer, Stealth BioTherapeutics  
- Vision Disorder Panel Q&A

11:40am  Mini-Break

11:45am  Pre-Clinical Data Updates  
Philip Yeske, PhD  
- KL1333 - Mitochondrial Disease Treatment Opportunity by NAD+ Modulation and Mitochondrial Biogenesis  
  Magnus Hansson, PhD, Chief Medical Officer, NeuroVive Pharmaceuticals
12:00pm  Planning an Externally-led Patient-Focused Drug Development Meeting
- EL-PFDD Meeting Background (James Valentine)
- EL-PFDD Mitochondrial Disease Plan (Philip Yeske, PhD)
- EL-PFDD Meeting Timeline and Q&A

12:30 pm  Lunch

Afternoon Platform Session (6)  Hermitage ABC
Targeting Mitochondria in Common Diseases

2:00 pm  Small Molecular Activators of Aldehyde Dehydrogenases - a New Treatment for Mitochondrial Dysfunction and Mitopathies?
Daria Mochly-Rosen, PhD, Stanford University, Stanford, CA

2:30 pm  Mitochondria and Diabetes
Gerald I. Shulman, MD, PhD, Yale University School of Medicine, New Haven, CT

3:00 pm  Neuroprotective Effects of Inhibition of the Mitochondrial Pyruvate Carrier
Anne N. Murphy, PhD, University of California San Diego, La Jolla, CA

3:30 pm  Break

3:45 pm  Medicines for Malaria and African Sleeping Sickness
Kiyoshi Kita, PhD, Nagasaki University, Japan

4:15 pm  UMDF Funded Projects
Philip Yeske, PhD, UMDF Science & Alliance Officer, Pittsburgh, PA

4:45 pm  Adjourn

6:00 pm  Reception

7:00 pm  Friday Night Banquet and Awards Ceremony
Day 4: Saturday, June 30, 2018

Morning Platform Session (7)  
*Platform:* Mitochondrial Stress Responses

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<tr>
<th>Time</th>
<th>Title</th>
<th>Speaker(s)</th>
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<tbody>
<tr>
<td>8:00 am</td>
<td>Mitochondrial Disease Sequence Data Resource (MSeqDR)</td>
<td>Marni Falk, MD, Children’s Hospital of Philadelphia, Philadelphia, PA</td>
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<td>and Colleen Clarke Muraresku, CGC</td>
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<td>9:00 am</td>
<td>Mitochondria Drug Toxicity and the Pharmaceutical Industry</td>
<td>Yvonne Will, PhD, Pfizer R&amp;D, Groton, CT</td>
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<td>9:30 am</td>
<td>Compensatory Responses to Mitochondrial Toxicity</td>
<td>Kendall B. Wallace, PhD, University of Minnesota Medical School, Duluth, MN</td>
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<td>10:00 am</td>
<td>Break</td>
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<tr>
<td>10:30 am</td>
<td>Manipulating Mitochondrial ROS and Oxidative Damage as Therapeutic Strategies</td>
<td>Mike Murphy, PhD, MRC Mitochondrial Biology Unit, University of Cambridge, UK</td>
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<td>11:00 am</td>
<td>Stress Responses in Mitochondrial Disease</td>
<td>Anu Suomalainen, MD, PhD, University of Helsinki, Finland</td>
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<td>11:30 am</td>
<td>Closing</td>
<td>Vamsi K. Mootha, MD</td>
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