Mitochondrial Medicine 2017: Washington DC

Scientific Program Schedule

Day 1: Wednesday, June 28, 2017

Morning Session
Platform: Mitochondria Division, Dynamics, Mitophagy

8:00 am  Welcome
William Copeland, PhD, and Michio Hirano, MD, Course Co-Chairs

8:15 am  AMPK: Restoring Metabolic Balance and Mitochondrial Homeo
Reuben J. Shaw, PhD

8:45 am  Damage Control: How PINK1 And Parkin Survey Mitochondrial Fidelity And
Respond With Selective Autophagy
Richard J. Youle, PhD

9:15 am  Abstract Presentations

<table>
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<tr>
<th>MMS Workshop Sessions</th>
<th>Location: Beech</th>
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<tr>
<td>9:30 am  Standards of Care and “Best Practices” in Mitochondrial Medicine</td>
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<tr>
<td>Sumit Parikh, MD</td>
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<td>10:15 am  Centers of Excellence Guidelines and Implementation Strategies</td>
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<td>Amy Goldstein, MD</td>
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<td>11:15 am  Break</td>
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<td>11:30 am  MSeqDR Workshop</td>
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<td>Marni Falk, MD; Colleen Clarke-Muraresku, MS, CGC; and Elizabeth M. McCormick, MS, CGC</td>
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<td>9:45 am  Break</td>
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<td>10:15 am  The Mechanisms and Implications of Mitochondrial Antigen Presentation</td>
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<td>Heidi McBride, PhD</td>
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<td>10:45 am  Abstract Presentations</td>
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<td>12:00 pm  Late Breaking - Drug Repurposing to Identify Drugs that Modify Mitochondrial Diseases</td>
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<td>Gino Cortopassi, PhD</td>
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<td>12:30 pm  Lunch</td>
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Day 2: Thursday, June 29, 2017

Afternoon Session
Platform: Mitochondrial RNA Processing and Transactions

2:00 pm  Characterization of Mammalian PNPase and its Role in Mitochondrial Biogenesis
Carla Koehler, PhD

2:30 pm  RNaseH1: Primer Removal, Human Mutation and Dual Localization
Robert Crouch, PhD

3:00 pm  Abstract Presentations

3:30 pm  Break

4:00 pm  Defining the Molecular Basis of Mitochondrial Disease
Robert W. Taylor, PhD, DSc, FRCPa

4:30 pm  Abstract Presentations

5:30 pm  Break & Posters Sessions (Non-CME) and Reception
Even Numbers Stay at Posters

7:30 pm  Adjourn

Morning Session
Platform: Mitochondria in Aging and Age-Related Diseases

8:00 am  Late Breaking - Effects of Elamipretide in Adults with Primary Mitochondrial Myopathy: a Phase 2 Double-Blind, Randomized, Placebo-Controlled Crossover Trial
Amel Karaa, MD

8:15 am  Powering Senescence: The Ugly Side of Mitochondria
Joao Passos, PhD

8:45 am  Role of Mitochondria-Associated ER Membranes in Alzheimer Disease
Eric A. Schon, PhD

9:15 am  In Vitro and In Vivo Studies of the ALS-FTLD Mitochondrial Protein CHCHD10
Giovanni Manfredi, MD, PhD

9:45 am  NAMDC Update (Non CME)
Michio Hirano, MD

10:00 am  Abstract Presentations
Day 3: Friday, June 30, 2017

**Schedule**

**Morning Session**

*Platform: Therapies...Genetics, Nucleotide, Research*

- **8:00 am** Mitochondrial Donation  
  Doug Turnbull, MBBS, MD, PhD, FRCP, FMedSci

- **8:30 am** Nucleotide Therapy  
  Michio Hirano, MD

**Afternoon Session**

*Platform: Mitochondrial Lipids and Barth Syndrome*

- **2:00 pm - 6:00 pm** NIH Young Investigator Workshop  
  Location: Beech

  - **2:00 pm** Clinical Aspects of Barth Syndrome  
    Hilary Vernon, MD, PhD

  - **2:30 pm** Cardiolipin and Barth Syndrome  
    Michael Schlame, MD

  - **3:00 pm** Molecular Physiology of Barth Syndrome  
    William T. Pu, PhD

  - **3:30 pm** Break

  - **4:00 pm** Cardiolipin Interactions with Mitochondrial Proteins  
    Anna Duncan, PhD

  - **4:30 pm** Molecular Basis for the Exchange of Phospholipids at Endoplasmic Reticulum to Mitochondria Contact Sites  
    Pascal F. Egea, PhD

*Platform: Late-Breaking Science*

- **5:00 pm** Human Mitochondrial Ribosomes Can Switch their Structural RNA Composition  
  Zofia Chrzanowska-Lightowlers, PhD

- **5:30 pm** Mechanisms of Maternal Mitochondrial Inheritance  
  Ding Xue, PhD

- **6:00 pm** Adjourn

*Attention: NEW Schedule - The Children’s Hospital Medical Center of Akron designates this live activity for a maximum of 20.50 AMA PRA Category I Credits™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.*
9:00 am  Mitochondria and Autism -- A Small Phase I/II Clinical Trial of Antipurinergic Therapy in Autism  
Robert K. Naviaux, MD, PhD

9:30 am  Advances on the Use of Designer Nucleases to Reduce Mutant mtDNA Levels  
Carlos T. Moraes, PhD

10:00 am  Succinate Prodrugs that Bypass Complex I  
Johannes K. Ehinger, MD, PhD

10:30 am  Break

11:00 am  Abstract Presentations and UMDF Funded Projects  
Philip Yeske, PhD and 2017 UMDF Funded Researchers

12:30 pm  Lunch

Afternoon Session
Platform: Role of Mito in Inflammation and Nuclear Cross Talk

2:00 pm  Mitochondrial Stress Signaling in Disease, Aging, and Immunity  
Gerald S. Shadel, PhD

2:30 pm  The Mitochondrial UPR and Pathogenic Bacteria  
Cole Haynes, PhD

3:00 pm  New Targets to Stimulate Mitochondria in Immunity  
Marcia Haigis, PhD

3:30 pm  Break

4:00 pm  Mitochondrial and Nuclear DNA Intertalk Shapes Organismal Metabolism  
Jose Antonio Enríquez, PhD

4:30 pm  Adjourn

6:00 pm  Reception

7:00 pm  Banquet & Awards Ceremony  
Keynote: Christopher P. Austin, MD  
Director, National Center for Advancing Translational Sciences, National Institutes of Health

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Day 4: Saturday, July 1, 2017

Morning Session
Platform: Clinical Trials and What’s on the Horizon

8:00 am  UMDF Updates, MCDR Patient Registry, and Roadmap to a Cure
Philip Yeske, United Mitochondrial Disease Foundation (Non CME)

8:30 am  Clinical Trials for Mitochondrial Disease - Updates (Non CME)

8:30 am  Update from BioElectron
Matthew Klein, BioElectron Technology Corporation (Non CME)
(formerly Edison Pharmaceuticals)

8:55 am  Stealth BioTherapeutics Update
Reenie McCarthy, Stealth BioTherapeutics (Non CME)

9:20 am  Rationale and Design of a Clinical Study of Omaveloxolone in Patients with Mitochondrial Myopathies
Edmund Doherty, Reata Pharmaceuticals (Non CME)

9:40 am  LEROS - a long term, multicenter, open-label clinical study using idebenone in LHON
Xavier Lloria, Santhera Pharmaceuticals (Non CME)

10:00 am Coffee Break

10:30 am  Open Clinical Studies (Non CME)
Kris Engelstad, NAMDC and Columbia University

11:00 am  Abstract Presentations (CME)

11:45 am  Summary of International Clinical Trial Readiness Meetings
Michio Hirano, MD (CME)

12:00 pm  A Converging Mitochondrial Pathway D destructed in Parkinson’s Disease
Xinnan Wang, MD, PhD (CME)

12:30 pm  Adjourn and Box Lunch

Attention: NEW Schedule - The Children’s Hospital Medical Center of Akron designates this live activity for a maximum of 20.50 AMA PRA Category I Credits™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.