2016 Course Description

The United Mitochondrial Disease Foundation, the Mitochondria Research Society, the Mitochondrial Medicine Society, the Mitochondrial Physiology Society, and Children’s Hospital Medical Center of Akron (CHMCA) have joined efforts to sponsor and organize another CME-accredited symposium. Mitochondrial diseases are more common than previously recognized and mitochondrial pathophysiology is now a recognized part of many disease processes, including heart disease, cancer, AIDS and diabetes. There have been significant advances in the molecular genetics, proteomics, epidemiology and clinical aspects of mitochondrial pathophysiology. This conference is directed toward the scientist and clinician interested in all aspects of mitochondrial science. The content of this educational program was determined by rigorous assessment of educational needs and includes surveys, program feedback, expert faculty assessment, literature review, medical practice, chart review and new medical knowledge. The format will include didactic lectures from invited experts intermixed with peer-reviewed platform presentations. There will be ample time for professional discussion both in and out of the meeting room, and peer-reviewed poster presentations will be given throughout the meeting. This will be a four-day scientific meeting aimed at those with scientific and clinical interests.

Learning Objectives

At the completion of the course, attendees will:

- Recognize the ways in which animal models of mitochondrial disease can help us better understand mitochondrial disease mechanisms in humans and develop new concepts in the pathophysiology of human mitochondrial disease as well as therapeutic interventions.
- Illustrate how mtDNA replication and stability is a key factor in mitochondrial health and how impaired mtDNA replication and translation can lead to mitochondrial disease in humans.
- Explain the current knowledge of mitochondrial diseases of the eyes and eye movement, focusing on those diseases that lead to visual loss and optic atrophy as well as ophthalmoplegia.
- Describe the new therapeutic techniques in altering mitochondrial function, including mitochondrial replacement therapy and the use of zinc-finger nucleases, mitoTALENs and CRISPER technology.
- Compare the lessons learned with other rare diseases in terms of therapeutic drug development and how to partner with the FDA for clinical trial design.
- Describe the ways in which cellular nutrients and nutritional interventions influence mitochondrial function and clinical trial design.
- Summarize the other functions of the mitochondria, including the cell-danger response and how external toxins affect mitochondrial function.
- Distinguish how patient priorities, new drugs and personalized clinical trials are being aligned to bring effective treatments to mitochondrial disease patients.

Target Audience

Researchers/Scientists, Neurologists, Geneticists, Pediatrics, Internal Medicine, Nephrologists, Cardiologists, Endocrinologists, Genetic Counselors, Advanced Practice Nurses, Physicians Assistants, RNs, Occupational Therapy, Physical Therapy and Speech-Language Pathology, Nutritional Therapy, and Residents/Fellows/Students

Credits

The Children’s Hospital Medical Center of Akron designates this live activity for a maximum of 25.25 AMA PRA Category I Credits™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.
Wednesday, June 15, 2016

Morning Session

*Platform: Use of Model Systems - Primarily Mouse*

8:00 am
Welcome
Russell Saneto, DO, PhD, Course Chair
Seattle Children’s Research Institute

8:15 am
Complex I Function from Worms to Man
Philip Morgan, MD
Seattle Children’s Research Institute

8:45 am
Mouse and Cellular Models of Mitochondrial Complex I Deficiency
David Thorburn, PhD
Murdoch Children’s Research Institute

9:15 am
Dissecting Neuronal Susceptibility in a Mouse Model of Leigh Syndrome
Albert Quintana, PhD
Universitat Autònoma de Barcelona

9:45 am Break

10:15 am
Interventions that Alleviate Severe Mitochondrial Disease in the Ndufs4 Knockout Mouse
Matt Kaeberlein, PhD
University of Washington

10:45 am Break

11:00 am
Mitochondrial DNA Replication and Transcription
Maria Falkenberg, PhD
University of Gothenberg

11:30 am Abstract Presentations

12:15 pm Lunch

Afternoon Session

*Platform: DNA Replication and Transcription*

2:00 pm
Regulation of Mitochondrial Transcription by TFAM-directed, Sequence-specific mtDNA Looping
Craig Cameron, PhD
Pennsylvania State University

2:30 pm
Mouse and Cellular Models of Mitochondrial Complex I Deficiency
David Thorburn, PhD
Murdoch Children’s Research Institute

3:00 pm Break

3:30 pm
Replication-Transcription Switch in Human Mitochondria
Dmitry Temiakov, PhD
Rowan University

4:15 pm
Abstract Presentations

5:45 pm
Break & Posters Sessions (Non CME)
Even Numbers Stay at Posters

7:30 pm Adjourn

Thursday, June 16, 2016

Morning Session

*Platform: Mitochondria and the Eye*

8:00 am
Using the Eye to Peer at the Brain - New Tools
Alfredo Sadun, MD
University of California Los Angeles

8:30 am
Mitochondrial Optic Neuropathies-Clinical Manifestations and Natural History
Patrick Yu-Wai-Man, MD, PhD
Newcastle University

9:00 am
New Optic Atrophy Genes and Mechanistic Insights
Guy Lenaers, MD
Mitochondrial Medicine Research Center

9:30 am
Eye Movements at the Interface of Muscle and Brain Energy Pathways
Avery Weiss, MD
Seattle Children’s Hospital

10:00 am Break

10:30 am
Animal Models of Mitochondrial Disease: Platforms for Therapeutic Development
Douglas C. Wallace, PhD
Children’s Hospital of Philadelphia

11:00 am
Abstract Presentations

12:15 pm Lunch

Afternoon Session

*Platform: Novel Methods of Altering Mitochondrial Function*

2:00 pm
Mitochondrial Replacement Therapy
Shoukhrat Mitalipov, PhD
Oregon Health and Science University

2:30 pm
Mitochondrial Therapy of Lung Disease
Jahar Bhattacharya, MD, DPhil
Columbia University

3:00 pm
Modulating mtDNA Heteroplasmy with Designer Nucleases
Carlos T. Moraes, PhD
University of Miami
Friday, June 17, 2016
Morning Session
Platform: Study Design and Other Regulatory Issues Involving Clinical Trials for Mitochondrial Diseases

8:00 am
Welcome
Chuck Mohan, CEO and Patrick Kelley, Chairman
UMDF

8:30 am
Introduction of the Session
Peter Stacpoole, PhD, MD
University of Florida

9:00 am
Therapeutic Development in Cystic Fibrosis: Lessons Learned
Margaret Rosenfeld, MD, MPH
Seattle Children’s Research Institute

9:30 am
From the FDA: A Clinical Perspective
Dragos Roman, MD
Food and Drug Administration

10:00 am
FDA Perspective - Various Approaches to Developing Outcome Measures for Rare Disease Clinical Trials
Elektra Papadopoulos, MD
Food and Drug Administration

10:30 am Break

11:00 am
Achieving Meaningful Regulatory and Clinical Outcomes for Patients: Strategies in Rare Disease Therapy Development
Dave Schubert
Stealth Biotherapeutics

11:30 am
How Small Businesses and the Academic Community Partner to Address Funding
Rich Wagner, PhD, President and CE
Medosome Biotec, Ltd

12:00 pm
Panel Discussion
All speakers

12:30 pm Lunch

A five minute question and answer period is allotted for each presentation.

Saturday, June 18, 2016
Morning Session
Platform: Hot Topics in Mitochondrial Medicine

8:00 am
Mitochondrial Cell Danger Response
Robert K. Naviaux, PhD
University of California San Diego

8:45 am
Exposure-Related Mitochondrial Disease
Kendall Wallace, PhD
University of Minnesota

9:30 am
DNA Polymerase z Functions in Mitochondria
Keshav K. Singh, PhD
University of Alabama at Birmingham

10:30 am
Update on Edison Clinical Trials
Matthew B. Klein, MD, MS, FACS
Edison Pharmaceuticals

11:00 am Lunch
(Pick up box lunches and return to seating)

Afternoon Session
Platform: Optimizing Outcome Measures in Mitochondrial Disease Research Trials and Clinical Care – Mitochondrial Medicine Society (MMS)

11:30 am
Identifying Strategic Patient-centered Research Themes and Validated Outcomes
Grainne Gorman, PhD
Wellcome Trust Centre for Mitochondrial Research, UK

12:30 pm
Lessons Learned on the Forefront of Mitochondrial Drug Development
Matthew B. Klein, MD, MS, FACS
Edison Pharmaceuticals

1:30 pm
Clinical Trial Design – Digging in Deeper for Best Practices
TBD

2:30 pm
MSeqDR Hands-on Workshop (non CME)

4:00 pm Adjourn
2016 Scientific Planning Committee

- Russell Saneto, DO, PhD - Course Chair
  Seattle Children's Research Institute, Seattle, WA
- Bruce H. Cohen, MD - 2016 CME Chair
  Akron Children's Hospital, Akron, OH
- William Copeland, PhD
  NIEHS Research, Triangle Park, NC
- Marni Falk, MD
  The Children's Hospital of Philadelphia, PA
- Amy Goldstein, MD
  Children's Hospital of Pittsburgh, Pittsburgh, PA
- Carla Koehler, PhD
  University of California Los Angeles, Los Angeles, CA
- Matthew Klein, MD, MS, FACS
  Edison Pharmaceuticals, Mountain View, CA
- Danuta Krotoski, PhD
  Eunice Kennedy Shriver National Institute of Child Health and Human Development, NIH, Bethesda, MD
- Giovanni Manfredi, MD, PhD
  Weill Medical College of Cornell Univ, New York, NY
- Philip Morgan, MD
  Seattle Children's Research Institute, Seattle, WA
- Robert K. Navaux, MD, PhD
  University of California San Diego, San Diego, CA
- Sumit Parikh, MD
  The Cleveland Clinic, Cleveland, OH
- Margaret Sedensky, MD
  Seattle Children's Research Institute, Seattle, WA
- Peter Stacpoole, PhD, MD
  University of Florida, Gainesville, FL
- Keshav K. Singh, PhD
  University of Alabama at Birmingham, AL
- Kara Strittmatter, CMM, MA
  UMDF Meeting Event Director, Pittsburgh, PA
- Philip Yeske, PhD
  UMDF Science & Alliance Officer, Pittsburgh, PA

2016 Scientific Meeting Faculty

- Margaret Rosenfeld, MD
  Seattle Children's Research Institute, Seattle, WA
- Keshav K. Singh, PhD
  University of Alabama at Birmingham, AL
- Peter W. Stacpoole, PhD, MD
  University of Florida, Gainesville, FL
- Dmitry Temiakov, PhD
  Rowan University, Stratford, NJ
- David Schubert
  Stealth Biotherapeutics, Newton, MA
- Keshav K. Singh, PhD
  University of Alabama at Birmingham, AL
- Peter W. Stacpoole, PhD, MD
  University of Florida, Gainesville, FL
- Elektra Papadopoulous, MD, MPH
  Food and Drug Administration, Silver Spring, MD
- Robert K. Navaux, MD, PhD
  University of California San Diego, San Diego, CA
- David Schubert
  Stealth Biotherapeutics, Newton, MA
- Keshav K. Singh, PhD
  University of Alabama at Birmingham, AL
- Peter W. Stacpoole, PhD, MD
  University of Florida, Gainesville, FL
- Elektra Papadopoulous, MD, MPH
  Food and Drug Administration, Silver Spring, MD
- Albert Quintana, PhD
  Universitat Autònoma de Barcelona, Spain
- Margaret Rosenfeld, MD, MPH
  Seattle Children's Research Institute, Seattle, WA
- Alfredo A. Sadun, MD, PhD
  University of California Los Angeles, Los Angeles, CA

ACREDITATION STATEMENT
This activity has been planned and implemented in accordance with the accreditation requirements and policies of the Ohio State Medical Association (OSMA) through the joint providership of Children's Hospital Medical Center of Akron and The United Mitochondrial Disease Foundation. The Children's Hospital Medical Center of Akron is accredited by the OSMA to provide continuing medical education for physicians.
SCIENTIFIC PROGRAM REGISTRATION FORM
Mitochondrial Medicine 2016: Seattle
DoubleTree by Hilton Seattle Airport
June 15 - 18, 2016

REGISTER NOW TO GUARANTEE YOUR ATTENDANCE!

Four Ways to Register:
1. Complete the registration form below and mail it back to the UMDF.
2. Complete the registration form below and fax it to UMDF at 412-793-6477.
3. Register online at www.umdf.org/symposium/registration. Use the registration code SCI1602.
4. Scan the QR code located below and register online. Use the registration code SCI1602.

Registration Code: SCI1602

FEES (Full four-day registration) - Please check only one
☐ $825  Physician/Researcher
☐ $775  UMDF/MMS/MRS/MIP Members (discount rate)
☐ $450  RNs, Genetic Counselors(GCs)**/
  Allied Health Professionals, Resident/Fellow*/Students

(* Letter from program director must be received in our office before May 29, 2016, in order to receive this fee.)
(** GCs - Register online for CEU credit)

Full registration entitles you to a hard copy syllabus, daily continental breakfast, refreshment breaks, lunch and the Friday night UMDF banquet. Daily Rate Fee includes all of the above except for the banquet. Payment must be received prior to attendance at symposium.

Total Full Registration Fee: $ ________________________

Can’t attend all four days? Choose the days that you can attend with our daily rate.

We offer a $200 daily rate for Scientific/Medical Sessions. Check all that apply.

☐ Wednesday, June 15  ☐ Thursday, June 16  ☐ Friday, June 17  ☐ Saturday, June 18

# ____________ of days X $200 = TOTAL $ ________________

I would also like to attend the UMDF Friday Night Banquet (included in a four day registration)

# ____________ of people X $75 = TOTAL $ ________________

Total Daily Rate Fees Included   $____________________

Please make all checks payable to: The United Mitochondrial Disease Foundation

Card Number ____________________________________________   Expiration Date _____________

Name as listed on card (please print)  _____________________________________________________

Signature ______________________________________________________ (invalid without signature)

Special Dietary Requirements ☐ Vegetarian ☐ Gluten-Free ☐ Other __________________

(Contact hotel directly prior to arrival to confirm)

Deadline for mail-in registration: May 29, 2016
Deadline for online registration: June 9, 2016
Registrations will only be accepted on-site after these dates.

Fees
Late and On-Site Registration (Full): $100
Late and On-Site Registration (Daily): $25/day

All cancellations will be subject to a service charge based on registration fee amounts – a small percentage must be deducted to cover processing charges to the foundation. A written notification of cancellation must be made to UMDF in order to process any refunds. No refunds will be issued after Friday, June 26, 2016.

Deadline

Mail to UMDF, 8085 Saltsburg Road, Suite 201, Pittsburgh, PA 15239 or fax to 412-793-6477
Mitochondrial Medicine: 2016
Seattle, WA

June 15-18, 2016
DoubleTree by Hilton
Seattle Airport