



CONFERENCE LOCATION

Newport Beach Marriott Hotel & Spa
900 Newport Center Drive
Newport Beach, CA 92660
Reservations: 1-866-440-3375

The Newport Beach Marriott Hotel & Spa features newly redesigned rooms and is close to public transportation. John Wayne Airport is five miles from the hotel. Long Beach Airport is 25 miles from the hotel and Los Angeles International Airport is 44 miles from the hotel. The United Mitochondrial Disease Foundation (UMDF) symposium rate is \$159 (USD)/per night plus tax. To reserve your room online, visit www.umdf.org/symposium/conferencelocation or call 1-866-440-3375. BOOK EARLY - OUR ROOM BLOCK FILLS UP QUICKLY. GROUND TRANSPORTATION INFORMATION is also available on the website.

Travel scholarships are available for new researchers. Visit www.umdf.org/symposium for more information.

2013 Scientific Planning Committee

Gregory M. Enns, MB, ChB, 2013 Course Chair
Stanford University Medical Center, Palo Alto, CA
Vamsi Mootha, MD, 2012 Outgoing Course Chair
Harvard Medical School, Boston, MA
Bruce H. Cohen, MD, 2013 CME Chair
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University of Florida, Gainesville, FL
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The Cleveland Clinic, Cleveland, OH
Keshav Singh, PhD
University of Alabama, Birmingham, AL
Peter Stacpoole, PhD, MD
University of Florida, Gainesville, FL

2013 Scientific Meeting Faculty

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Stanford Cancer Institute, Palo Alto, CA
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California Institute of Technology, Pasadena, CA
Paul Coates, PhD
Office of Dietary Supplements, NIH, Bethesda, MD
Bruce H. Cohen, MD
Akron Children's Hospital, Akron, OH
Salvatore DiMauro, MD
Columbia University Medical Center, New York, NY
Gregory M. Enns, MB, ChB
Stanford University Medical Center, Palo Alto, CA
Marni J. Falk, MD
Children's Hospital of Philadelphia, Philadelphia, PA

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Phil Morgan, MD
Seattle Children's Hospital, Seattle, WA
Thomas O'Brien, PhD
University of Florida, Gainesville, FL
Juan Pascual, MD, PhD
University of Texas Southwestern Medical Center, Dallas, TX
Jeanine Prompers, PhD
Eindhoven University of Technology, Eindhoven, Netherlands
Rodrigue Rossignol, PhD
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Ann Saada (Reisch), PhD
Hadassah-Hebrew University Medical Center, Jerusalem, Israel
Russell Saneto, DO, PhD
*Seattle Children's Hospital
University of Washington, Seattle, WA*
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Cardero Therapeutics, San Francisco, CA
Stuart J. Swiedler, MD, PhD
BioMarin, Oakland, CA
LaRee A. Tracy, MA, PhD, CDR
U.S. Public Health Service, San Diego, CA
Douglas Wallace, PhD
Children's Hospital of Philadelphia, Philadelphia, PA
Travis Wilson, CEO
Stealth Peptides, Inc., Newton Centre, MA

REGISTER NOW!

Mitochondrial Medicine: 2013
*Mitochondrial Therapeutics
From Bench To Bedside*
June 12-15, 2013
Newport Beach Marriott
Hotel and Spa
Newport Beach, CA



2013 Course Chair - Gregory M. Enns, MB, ChB
2013 CME Chair - Bruce H. Cohen, MD
Register now at www.umdf.org/symposium



Mitochondrial Medicine: 2013
Mitochondrial Therapeutics - From Bench To Bedside
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Pittsburgh, PA 15239



Mitochondrial Medicine: 2013
Mitochondrial Therapeutics-From Bench To Bedside



2013 Course Description

The United Mitochondrial Disease Foundation, the Mitochondrial Medicine Society, the Mitochondria Research Society, the Mitochondrial Physiology Society, and Northeast Ohio Medical University have joined efforts to sponsor and organize another multi-disciplinary 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.

Credits

Northeast Ohio Medical University designates this live activity for a maximum of 23.0 AMA PRA Category I Credits™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

Learning Objectives

At the completion of the course the attendees will:

- Be able to define the current state of knowledge of mitochondrial DNA mutations and nuclear DNA mutations as they result in human disease, focusing on the current and future state of NextGen genetic testing
- Have an understanding of the current state of knowledge of the pathogenic mechanisms of mitochondrial diseases and the impact of the modern pharmacopeia on mitochondrial function and dysfunction
- Become knowledgeable of the evolving field of mitochondrial proteomics, including detailed focus on the mitochondrial ribosomes and the mitochondrial polymerase and systems biology as it relates to mitochondrial physiology and disease
- Understand the current state of diagnostic testing for mitochondrial diseases
- Gain knowledge on mitochondrial biochemistry, physiology and dynamics and relate this to the pathophysiology of mitochondrial disease
- Understand the new pharmacological approaches to mitochondrial disorders and the mechanisms of how these new medications may work on improving mitochondrial energy production and reduction of oxidative stress



2013 SCIENTIFIC PROGRAM

Newport Beach Marriott Hotel & Spa
Newport Beach, California

June 12-15, 2013



Afternoon Platform Session
Platform Title: Next Gen Reporters of Cellular Metabolism

9:30 A.M. - 10:00 A.M.
Abstract Presentations

10:00 A.M. - 10:30 A.M. Break

10:30 A.M. - 11:00 A.M.
Targeting Drug Therapy To Cell Signaling Alterations
Marni J. Falk, MD
Children's Hospital of Philadelphia
Philadelphia, PA

11:00 A.M. - 12:15 P.M.
Abstract Presentations

12:15 P.M. Lunch

Afternoon Platform Session
Platform Title: Translational Mitochondrial Medicine

2:00 P.M. - 2:30 P.M.
Coenzyme Q10 as a Therapy for Mitochondrial Disease
Iain Hargreaves, PhD
National Hospital
London, United Kingdom

2:30 P.M. - 3:00 P.M.
The Role of Arginine and Citrulline Supplementation in MELAS Syndrome
Fernando Scaglia, MD, FACMG
Baylor College of Medicine
Houston, TX

3:00 P.M. - 3:30 P.M.
Diseases of Mitochondrial Translation
Rita Horvath, MD, PhD
Newcastle University
Newcastle upon Tyne, United Kingdom

3:30 P.M. - 3:45 P.M. Break

3:45 P.M. - 4:15 P.M.
Mitochondrial Function Ex Vivo – the Use of Fibroblasts
Ann Saada (Reisch), PhD
Hadassah-Hebrew University Medical Ctr.
Jerusalem, Israel

4:15 P.M. - 5:30 P.M.
UMDF 2013 Funded Projects Abstracts

5:30 P.M. Adjourn

6:00 P.M. Reception

7:00 P.M. Banquet & Awards Ceremony

2:00 P.M. - 2:30 P.M.
Mitoplasticity: Mitochondrial Adaption Biology – Implications for Therapies in Cancer and Neurodegenerative Diseases
Rodrigue Rossigol, PhD
Université Victor Segalen Bordeaux 2
Bordeaux, France

2:30 P.M. - 3:00 P.M.
Optical Sensors for In Vivo Biochemistry
Wolf B. Frommer, PhD
Carnegie Institute of Science
and Stanford School of Medicine
Palo Alto, CA

3:00 P.M. - 4:30 P.M.
Abstract Presentations

4:30 P.M. - 7:30 P.M.
Clinical Directors Workshop (Non CME)
Moderated by the Mitochondrial Medicine Society

4:30 P.M. - 6:30 P.M.
Break & Posters Sessions (Non CME)
Odd Numbers Stay at Posters
Reception and Cash Bar

6:30 P.M. Adjourn

Friday, June 14, 2013

Morning Platform Session
Platform Title: Animal Models and Translational Research

8:00 A.M. - 8:30 A.M.
Of Mice and Mitochondria
Douglas Wallace, PhD
Children's Hospital of Philadelphia
Philadelphia, PA

8:30 A.M. - 9:00 A.M.
Strategies for Determining Pathogenicity of POLG1 Variants
Laurie Kaguni, PhD
Michigan State University
East Lansing, MI

9:00 A.M. - 9:30 A.M.
Mitochondrial Biogenesis/Creatine
Carlos Moraes, PhD
Miami University
Miami, FL

Thursday, June 13, 2013

Morning Platform Session
Platform Title: Mitochondrial Imaging

8:00 A.M. - 8:30 A.M.
Regulation of Mitochondrial Dynamics
David Chan, MD, PhD
California Institute of Technology
Pasadena, CA

8:30 A.M. - 9:00 A.M.
Assessment of In Vivo Muscle Mitochondrial Metabolism by Magnetic Resonance Spectroscopy
Jeanine Prompers, PhD
Eindhoven University of Technology
Eindhoven, Netherlands

9:00 A.M. - 9:30 A.M.
Magnetic Resonance Spectroscopy Observation of Brain and Muscle
Juan M. Pascual, MD, PhD
University of Texas Southwestern Medical Ctr.
Dallas, TX

9:30 A.M. - 10:00 A.M.
HMPAO-SPECT – Redox Neuroimaging
Francis Blankenberg, MD
Stanford Cancer Institute
Palo Alto, CA

10:00 A.M. - 10:30 A.M. Break

10:30 A.M. - 12:00 P.M.
Abstract Presentations

12:00 P.M. Lunch

12:15 P.M. Lunch

Afternoon Platform Session
Platform Title: The Science Behind Clinical Management

2:00 P.M. - 2:30 P.M.
To Appreciate the Interactions of Anesthetics and Perioperative Medications with Mitochondrial Disease
Phil Morgan, MD
Seattle Children's Hospital
Seattle, WA

2:30 P.M. - 3:00 P.M.
It's Not Your Mother's Ribosome Anymore
Thomas O'Brien, PhD
University of Florida
Gainesville, FL

3:00 P.M. - 3:30 P.M.
Drugs – The Good, The Bad and The Ugly
Bruce H. Cohen, MD
Akron Children's Hospital
Akron, OH

3:30 P.M. - 4:15 P.M.
Abstract Presentations

4:15 P.M. - 6:30 P.M.
Break & Poster Sessions (Non CME)
Even Numbers Stay at Posters

6:30 P.M. Adjourn

Wednesday, June 12, 2013

Morning Platform Session
Platform Title: Mitochondrial Disorders in the 21st Century – Is Treatment Possible?

8:00 A.M. - 8:45 A.M.
Welcome and Treatment of Mitochondrial Disease
Gregory M. Enns, MB, ChB, Course Chair
Stanford University Medical Center
Palo Alto, CA

8:45 A.M. - 9:15 A.M.
NCATS - Repurposing Drugs For Rare Diseases
James Inglese, PhD
NCATS, NIH
Bethesda, MD

9:15 A.M. - 9:45 A.M.
NAMDC – Registries and the Future of Mitochondrial Trials
Salvatore DiMauro, MD
Columbia University
New York, NY

Russ Saneto, DO, PhD
Seattle Children's Hospital
Seattle, WA

9:45 A.M. - 10:15 A.M. Break

10:15 A.M. - 11:30 A.M.
Abstract Presentations

11:30 A.M. - 12:15 P.M.
Report on NIH Mitochondrial Disease initiatives and update on the MSeqDR Consortium
Marni Falk, MD
Children's Hospital of Philadelphia, PA

Danuta Krotoski, PhD
Eunice Kennedy Shriver National Institute of Child Health and Human Development, National Institutes of Health

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

SAVE TIME - REGISTER ONLINE at www.umdf.org/symposium

ACCREDITATION STATEMENT

This activity will be planned and implemented in accordance with the Essential Areas and Policies of the Accreditation Council for Continuing Medical Education (ACCME) and the Accreditation Council for Pharmacy Education (ACPE); through the co/joint sponsorship of Northeast Ohio Medical University and the United Mitochondrial Disease Foundation. Northeast Ohio Medical University is accredited by the ACCME and ACPE to provide continuing education for physicians and pharmacists.



SCAN THE QR CODE ABOVE ON YOUR SMART DEVICE TO REGISTER SECURELY NOW!



THE UMDF THANKS THE FOLLOWING FOR THEIR ASSISTANCE WITH THE PLANNING OF THIS MEETING



Registration and Cancellation

Mail in and online registrations will be accepted until Monday, June 3, 2013. After this date, registrations will only be accepted on site. Cancellations with a refund will only be made before Wednesday, June 5, 2013. All cancellations will be subject to a \$75 service charge. A written notification of cancellation must be made to UMDF in order to process your refund. No refunds will be issued after Friday, June 7, 2013.

SCIENTIFIC PROGRAM REGISTRATION FORM Mitochondrial Medicine 2013: Newport Beach Newport Beach Marriott Hotel & Spa - Newport Beach, CA June 12-15, 2013

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 code SCII302 when asked for your registration code.
4. Scan the QR code located on the left at the top of this page and register online. Use Code SCII302 when asked for your registration code.

Registration Code: SCII302

FEES (Full four-day registration) - Please check only one

- \$800 Physician/Researcher
- \$750 UMDF/MMS/MRS/MIP Members (discount rate)
- \$450 RN's/Allied Health Professionals/Residents/Fellows*/Students (*Letter from program director must be received in our office before June 3, 2013, in order to receive this fee.)

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. This year, the banquet will be outdoors. We suggest that you plan ahead in case of cool weather.

Total Full Registration Fee: \$ _____

* A \$100 late fee will be added to any full registrations received after May 28, 2013.

Can't attend all four days? Choose the days that you can attend with our daily rate. We offer a \$175 daily rate for Scientific/Medical Sessions. Check all that apply.

Wednesday, June 12 Thursday, June 13 Friday, June 14 Saturday, June 15

_____ of days X \$175 = 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

Please charge this registration to the following: Visa MC Disc AmEx
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Special Dietary Requirements Vegetarian or other _____

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Mail to UMDF, 8085 Saltsburg Road, Suite 201, Pittsburgh, PA 15239 or fax to 412-793-6477