



UMDF Power Surge 2020

June 26, 2020 – Clinical/Scientific/Family

June 27, 2020 – Patient/Family

On-Line Virtual Meeting

Day 1: Friday, June 26 – Clinical Trials Program - Scientific/Clinical/Patients

(All times are U.S. Eastern)

10:00 am UMDF Welcome

Brian Harman, UMDF President & CEO

10:10 am Clinical Trials – Nuts, Bolts and the Importance of Trials for Therapies

Frank J. Sasinowski, MS, MPH, JD, Hyman, Phelps & McNamara, Washington DC

10:40 am Overview of the Day - Panel Discussion of our Learnings

Philip Yeske, PhD, UMDF Science & Alliance Officer

10:45 am Panel 1 – FDA Workshop and Other Regulatory Learnings

Moderator: *Philip Yeske, PhD, UMDF Science & Alliance Officer*

Panelists: *Amel Karaa, MD, Massachusetts General Hospital/Harvard Medical School, Boston, MA; Patroula Smpokou, MD, Food and Drug Administration (FDA) - Division of Rare Diseases & Medical Genetics (DRDMG), Washington DC; and Frank J. Sasinowski, MS, MPH, JD, Hyman, Phelps & McNamara, Washington DC*

11:20 am Panel 2 – Patient Perspectives: Patient and Clinical Study Coordinator Panel

Moderator: *Philip Yeske, PhD, UMDF Science & Alliance Officer*

Panelists: *Monica Chulewicz, Adult Patient; Michael Hall, Parent/Caregiver; Lilly Lee, Clinical Research Coordinator, University of California San Diego, La Jolla, CA; Jessica Reit, MPH, Clinical Research Coordinator, University of California San Diego, La Jolla, CA; and Kristin Engelstad, MS, CGC, Clinical Study Coordinator, Columbia University, New York, NY*

11:55 am Panel 3 – Lessons Learned: Industry Panel

Moderators: *Amel Karaa, MD, Massachusetts General Hospital/Harvard Medical School, Boston, MA, and Philip Yeske, PhD, UMDF Science & Alliance Officer*

Panelists: *Jim Carr, PharmD, Stealth BioTherapeutics; Colin Meyer, MD, Reata Pharmaceuticals; Matthew Klein, MD, MS, FACS, PTC Therapeutics; and Patrice Rioux, PhD, formerly Raptor*

12:55 pm Break

Day 1: Friday, June 26 – Clinical Trials Program - Scientific/Clinical/Patients (cont'd)

1:15 pm Clinical Trial Updates

Speakers: Joanne Quan, MD, Modis/Zogenix; Tolga Uz, MD, PhD, Astellas Pharma; Chad Glasser, PharmD, MPH, Cycleron; and Alex Dorenbaum, MD, Reneo Pharmaceutical

Speakers: Michio Hirano, MD, Columbia University, New York, NY (Entrada Therapeutics MNGIE Natural History Study Lead Investigator); Magnus Hansson, MD, PhD, Abliva (formerly NeuroVive); Matthew Klein, MD, PhD, PTC Therapeutics; Noa Sher, PhD, Minovia Therapeutics; Magali Tael, MD, Gensight-Biologics; Peter W. Stacpoole, PhD, MD, University of Florida, Gainesville, FL; and Fernando Scaglia, MD, Baylor College of Medicine, Houston, TX

2:50 pm Panel 4 – Current Trials and Moving Forward – Primary Mitochondrial Myopathy Case Study

Moderators: Amel Karaa, MD, Massachusetts General Hospital/Harvard Medical School, Boston, MA, and Philip Yeske, PhD, UMDF Science & Alliance Officer

Panelists: Industry Representatives – Niall O'Donnell, Reneo Pharmaceutical; Magnus Hansson, MD, PhD, Abliva (formerly NeuroVive); and Tolga Uz, MD, PhD, Astellas Pharma

3:35 pm Impact of COVID-19 and Mitochondrial Medicine

Peter McGuire, MS, MD, The National Institute of Health, National Human Genome Research Institute, Bethesda, MD, and Eliza Gordon-Lipkin, MD, The National Institute of Health, National Human Genome Research Institute, Bethesda, MD

4:20 pm UMDF Collaborative Initiatives - North American Mitochondrial Disease Consortium (NAMDC), Mitochondrial Care Network (MCN), Mitochondrial Sequence Data Resource (MSeqDR), and Mitochondrial Disease Community Registry (MDCR)

NAMDC - Michio Hirano, MD, Columbia University, New York, NY; **MCN** - Sumit Parikh, MD, Cleveland Clinic, Cleveland, OH; **MSeqDR** - Marni Falk, MD, Children's Hospital of Philadelphia, Philadelphia, PA; and **MDCR** - Philip Yeske, PhD, UMDF Science & Alliance Officer

5:20 pm Closing Remarks

Day 2: Saturday, June 27 – Patient/Family Program

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- 10:00 am** **Welcome: UMDF and the Mitochondrial Medicine Community**
Brian Harman, UMDF President & CEO
- 10:30 am** **COVID-19: Managed Care and Urgent Care for Mitochondrial Patients**
Speaker: *Austin Larson, MD, Children's Hospital Colorado, Aurora, CO*
- 11:30 am** **Medications for Primary vs. Secondary Mitochondrial Disease in the Era of COVID-19**
Speakers: *Amel Karaa, MD, Massachusetts General Hospital/Harvard Medical School, Boston, MA; Amy Goldstein, MD, Children's Hospital of Philadelphia, Philadelphia, PA; and Sumit Parikh, MD, Cleveland Clinic, Cleveland, OH*
- 12:30 pm** **accelerators Brown Bag Big Pitch**
Speakers: *Kinsley Christopher Belle, Stanford University, Stanford, CA, and Lia Mayorga, Instituto de Histologia y Embriologia de Mendoza, Mendoza, Argentina*
- 1:30 pm** **Riding Out the COVID-19 Storm – Navigating the “New Normal”**
Speakers: *Margaret Moore, UMDF Support/Education Associate, and Jaclyn Leit, LCSW, BC-TMH, Denville, NJ*
- 2:30 pm** **Telehealth – Before, During and After COVID-19**
Speakers: *Amel Karaa, MD, Massachusetts General Hospital/Harvard Medical School, Boston, MA; Bruce Cohen, MD, Akron Children's Hospital, Akron, OH; and Sumit Parikh, MD, Cleveland Clinic, Cleveland, OH*
- 3:30 pm** **Closing Remarks**