



Externally-led Patient-Focused Drug Development Meeting

Friday, March 29, 2019

Program Agenda

7:30 am – 8:30 am Registration and Continental Breakfast

Opening Remarks and Morning Overviews

8:30 am – 8:40 am **Welcome**
Brian Harman, President & CEO, United Mitochondrial Disease Foundation

8:40 am – 8:50 am **Opening Remarks**
U.S. Food and Drug Administration (FDA), DNP - TBD

8:50 am – 9:05 am **Clinical Overview of Mitochondrial Myopathy in Adults**
Michio Hirano, MD, Columbia University, New York, NY

9:05 am – 9:15 am **Introduction, Overview of Meeting, and Audience and Remote Demographic Polling for Adults with Mitochondrial Myopathies**
James Valentine, JD, MHS, Meeting Moderator

Morning Session: Mitochondrial Myopathy - Adult Patient Perspective on the Burdens of the Disease and Current and Future Approaches to Treatments

9:15 am – 10:25 am **Panel #1 – Symptoms and Daily Impacts**

- Presentations by 5 Affected Individuals and Caregivers (25 minutes)
- Audience & Remote Polling Panel #1 (10 minutes)
- Moderated Audience Discussion Panel #1 (35 minutes)

10:25 am – 10:35 am Break (refreshments provided)

10:35 am – 11:45 am **Panel #2 – Current and Future Approaches to Treatments**

- Presentations by 5 Affected Individuals and Caregivers (25 minutes)
- Audience & Remote Polling Panel #2 (10 minutes)
- Moderated Audience Discussion Panel #2 (35 minutes)

11:45 am – 12:00 pm **Morning Session Closing Remarks**
Dr. Lucas Kempf, FDA, CDER Rare Diseases Program

12:00 pm – 1:00 pm Lunch Break

Afternoon Session: Neurologic Manifestations in Children with Mitochondrial Disease – Patient Perspective on the Burdens of the Disease and Current and Future Approaches to Treatments

1:00 pm – 1:05 pm **Afternoon Welcome and Overview**
Kira Mann, CEO, MitoAction and Brittany Hernandez, Director of Advocacy, Muscular Dystrophy Association (MDA)

1:05 pm – 1:20 pm **Clinical Overview: Neurologic Manifestations in Children with Mitochondrial Disease**
Amy Goldstein, MD, Children's Hospital of Philadelphia, PA

1:20 pm – 1:30 pm **Audience and Remote Polling – Attendee Demographics for Children with Neurologic Issues**

James Valentine, JD, MHS, Facilitator

1:30 pm – 2:40 pm **Panel #3 – Symptoms and Daily Impacts**

- Presentations by 5 Parents and Caregivers (25 minutes)
- Audience & Remote Polling Panel #3 (10 minutes)
- Moderated Audience Discussion Panel #3 (35 minutes)

2:40 pm – 2:50 pm **Break**

2:50 pm – 4:00 pm **Panel #4 – Current and Future Approaches to Treatment**

- Presentations by 5 Parents and Caregivers (25 minutes)
- Audience & Remote Polling Panel #4 (10 minutes)
- Moderated Audience Discussion Panel #4 (35 minutes)

4:00 pm – 4:15 pm **Afternoon Session Closing Remarks**

Larissa Lapteva, MD, MHS, MBA, FDA

4:15 pm – 4:45 pm **Next Steps and Closing Remarks**

Philip Yeske, PhD, UMDF Science & Alliance Officer and Brent Fields, UMDF Trustee Chair