UMDF Externally-led Patient-Focused Drug Development Sessions
Friday, March 29, 2019
Draft Agenda
NOTE: We will hold a final rehearsal on Thursday afternoon on March 28!

7:30 am – 8:30 am  Registration

8:30 am – 8:40 am  Opening Remarks & Meeting Overview – Brian Harman, UMDF CEO

8:40 am – 9:00 am  FDA Welcome – FDA - TBD

9:00 am – 9:15 am  Clinical Overview of Mitochondrial Myopathy in Adults – Amy Goldstein, MD

9:15 am – 9:25 am  Audience and remote polling – attendee demographics for Adults with Mitochondrial Myopathies – James Valentine

9:25 am – 9:50 am  Panel #1 - Adult Patient Perspective on the Burdens of the Disease (Focusing on Adults with Mito Myopathies)
• 9:25-9:30 - Laura Prudhomme-Gaupp (LA)
• 9:30-9:35 - Devin Shuman (PA)
• 9:35-9:40 - Deborah Bricker Parsons (GA)
• 9:40-9:45 - Rachel Schanzenbach (VA)
• 9:45-9:50 - Alyssa Delotte (FL)

9:50 am – 10:00 am  Audience and remote polling on Panel #1 questions

10:00 am – 10:30 am  Moderated audience discussion on Panel #1 questions

10:30 am – 10:40 am  Break (refreshments provided)

10:40 am – 11:05 am  Panel #2 - Adult Patient Perspective on Management of Care (Focusing on Adults with Mito Myopathies)
• 10:40-10:45 – Bob & Luisa Miller (IL)
• 10:45-10:50 – Deborah Carman (IL)
• 10:50-10:55 – Michael Monterio (MA)
• 10:55-11:00 – Sharon Shaw Reeder (AZ)
• 11:00-11:05 – Nicole DeJean (LA)

11:05 am – 11:15 am  Audience and remote polling on Panel #2 questions

11:15 am – 11:45 am  Moderated audience discussion on Panel #2

11:45 am – 12:00 pm  Summary of morning session and closing remarks – MDA Rep – PAG TBD
12:00 pm – 1:15 pm  Lunch Break

1:15 pm – 1:20 pm  Afternoon Welcome and Overview – MitoAction Rep, PAG TBD

1:20 pm – 1:35 pm  Clinical Overview: Neurologic Manifestations in Children with Mitochondrial Disease – Michio Hirano, MD

1:35 pm – 1:45 pm  Audience and remote polling – attendee demographics for children with neurologic issues – James Valentine

1:45 pm – 2:10 pm  Panel #3 - Parent Perspective on the Burdens of the Disease (Focusing on Pediatric/Youth Neurologic Issues)
   • 1:45-1:50 – Daniel Miller (CA)
   • 1:50-1:55 - Ann Korsen (NJ)
   • 1:55-2:00 - Annett Cotte (GA)
   • 2:00-2:05 - Stacy Taylor (MD)
   • 2:05-2:10 - Heather Thornbury (FL)

2:10 pm – 2:20 pm  Audience and remote polling on Panel #3 questions

2:20 pm – 2:50 pm  Moderated audience discussion Panel #3 questions

2:50 pm – 3:00 pm  Break

3:00 pm – 3:25 pm  Panel #4 - Parent Perspective on Management of Care (Focusing on Pediatric/Youth Neurologic Issues)
   • 3:00-3:05 - Carrie Mullin (NJ)
   • 3:05-3:10 - Lori Martin (TX)
   • 3:10-3:15 - Cheryl Porter (GA)
   • 3:15-3:20 - Gwen Lopez-Cohen (CT)
   • 3:20-3:25 - Anne Tuccillo (VA)

3:25 pm – 3:35 pm  Audience and remote polling on Panel #4 questions

3:35 pm – 4:05 pm  Moderated audience discussion on Panel #4 questions

4:05 pm – 4:20 pm  Summary of afternoon sessions, FMM Rep PAG TBD

4:20 pm – 4:40 pm  Closing Remarks from FDA – Larissa Lapteva, MD, MHS, MBA, U.S. Food and Drug Administration

4:40 pm – 4:45 pm  Closing Remarks and Next Steps - Philip Yeske, PhD, UMDF Science & Alliance Officer