

FDA Workshop on Developing Therapies for Primary Mitochondrial Diseases: Bridging the Gaps

September 6, 2019

8:00 am – 8:05 am	Welcome and Introduction, Dragos Roman, FDA
8:05 am – 8:30 am	Integrating Mitochondrial Biology into Designing Drug Development Programs, Robert Naviaux, UCSD
8:30 am – 9:00 am	Lessons Learned from Previous Drug Development Programs, Jim Carr, Stealth Biotherapeutics & Matthew Klein, BioElectron
9:00 am – 9:45 am	Leveraging Natural History Data when Designing Clinical Trials, Michio Hirano, Columbia University, Philip Yeske, UMDF & Anita Zaidi, FDA
9:45 am – 10:15 am	Patient Population Selection and Considerations for Pediatric Patient Enrollment in Clinical Trials, Amel Karaa, Massachusetts General Hospital & Melanie Bhatnagar, FDA
10:15 am – 11:00 am	Panel Discussion Moderated by Amel Karaa (Massachusetts General Hospital) with Robert Naviaux (UCSD), Matthew Klein (BioElectron), Irina Anselm (Boston Children’s Hospital), Kathrine Neveu (Patient Representative), Michio Hirano (Columbia University), Melanie Bhatnagar (FDA) & Patroula Smpokou (FDA)
11:00 am – 11:45 am	Open Discussion and Q&A
11:45 am – 1:00 pm	Lunch
1:00 pm – 1:45 pm	Defining and Assessing Clinical Benefits: Regulatory, Scientific, and Patient Perspectives, Sophia Hufnagel, FDA, Bruce Cohen, Akron Children’s Hospital & Phil Yeske, UMDF
1:45 pm – 2:15 pm	Panel Discussion Moderated by Philip Yeske (UMDF) with Andrea Gropman (Children’s National Health System), Mindy Leffler (Casimir Trials), Emily Milligan (Barth Syndrome Foundation), Jim Carr (Stealth Biotherapeutics), Ebony Dashiell-Aje (FDA), Teresa Buracchio (FDA/DNP), Dragos Roman (FDA) & Michio Hirano (Columbia University) & Rachel Witten (FDA)
2:15 pm – 2:45 pm	Clinical Trial Design and Statistical Considerations, Frank Sasinowski, Hyman, Phelps & McNamara, P.C. and Yan Wang, FDA

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| 2:45 pm – 3:15 pm | Panel Discussion Moderated by Alex Sherman (ALS Network and Massachusetts General Hospital) with Frank Sasinowski (Hyman, Phelps & McNamara, P.C.), Bruce Cohen (Akron Children’s Hospital), Tristan Massie, FDA, Yan Wang (FDA), Gregory Levin (FDA), Ilan Irony (FDA) & Jim Carr (Stealth Biotherapeutics) |
| 3:15 pm – 3:45 pm | Open Discussion and Q&A |
| 3:45 pm – 4:00 pm | Summary and Closing Remarks – who is doing this? |