



Primary Mitochondrial Diseases Workshop Agenda
(virtual public workshop)
May 22, 2025

10am	Welcome & Opening Remarks <ul style="list-style-type: none">Susan C. Winckler, RPh, Esq., Reagan-Udall Foundation for the FDA
10:05am	FDA Opening Remarks <ul style="list-style-type: none">Catherine Pilgrim-Grayson, MD, MPH, Center for Drug Evaluation and Research, FDA
10:10am	Setting the Stage: A Variety of Perspectives <ul style="list-style-type: none">Michio Hirano, MD, Columbia University Medical Center Clinical Trial Design and Implementation: Patient Population Considerations <ul style="list-style-type: none">Anna Choe, MD, MPH, Center for Drug Evaluation and Research, FDA Primary Mitochondrial Disease Drug Development <ul style="list-style-type: none">Reenie McCarthy, JD, Stealth BioTherapeutics
11:05am	Reactor Panel <ul style="list-style-type: none">Marni Falk, MD, Children's Hospital of Philadelphia & University of Pennsylvania Perelman School of MedicineBrian Tseng, MD, PhD, The POLG FoundationPhilip Yeske, PhD, United Mitochondrial Disease Foundation
11:35am	10-minute Break
11:45am	Selecting Patient-focused Outcomes & Statistical Considerations <ul style="list-style-type: none">Naomi Knoble, PhD, Center for Drug Evaluation and Research, FDAYan Wang, PhD, Center for Drug Evaluation and Research, FDA
12:15pm	Clinical Outcome Assessment Example <ul style="list-style-type: none">Amel Karaa, MD, Massachusetts General Hospital & Harvard Medical School
12:30pm	Reactor Panel <ul style="list-style-type: none">Kasey Woleben, Cure Mito FoundationZarazuela Zolkipli-Cunningham, MBChB, MRCP, Children's Hospital of Philadelphia & University of Pennsylvania Perelman School of Medicine
1:00pm	LUNCH

- 1:30pm** **Current Approaches, Challenges, and Opportunities**
- Chad Glasser, PharmD, MPH, Tisento Therapeutics
 - Magnus Hansson, MD, PhD, Abliva AB, A Member of Pharming Group
- Q&A (20 min)**
- 2:30pm** **Using What We've Learned To Move Forward**
- Jason Colquitt, Across Healthcare
 - Amel Karaa, MD, Massachusetts General Hospital & Harvard Medical School
 - Kerry Jo Lee, MD, Center for Drug Evaluation and Research, FDA
 - Sophia Zilber, Lived Experience Perspective
- 3:30pm** **Closing Remarks & Adjourn**