

MOVING FORWARD TOGETHER

EL-PFDD Agenda

May 28, 2024



9:00 - 9:05 AM	Welcome
9:05 - 9:30 AM	FDA Opening Remarks , Sarah Fuchs, MD, MSCI Clinical Overview and Background on the condition , William Rizzo, MD Overview of Discussion Format , Mousumi Bose, PhD Polling Questions
9:30 AM - 12:30 PM	Panel #1: Symptoms and Daily Impacts
9:30 - 9:40 AM	Vision and Hearing Sensory Loss Panel
9:40 - 9:55 AM	Polling and Large-Group Facilitated Discussion for Sensory Losses
9:55 - 10:05 AM	Mobility Panel
10:05 - 10:20 AM	Polling and Large-Group Facilitated Discussion for Mobility
10:20 - 10:30 AM	Intellectual Disabilities Panel
10:30 - 10:45 AM	Polling and Large-Group Facilitated Discussion for Intellectual Disabilities
10:45 - 10:55 AM	Break
10:55 - 11:10 AM	Seizures, GI, Respiratory and Other Systems Panel
11:10 - 11:25 AM	Polling and Large-Group Facilitated Discussion for Seizures, GI, Respiratory and Other Systems
11:25 - 11:35 AM	Leukodystrophy and Progression of the Disease Panel
11:35 - 11:50 AM	Polling and Large-Group Facilitated Discussion for Leukodystrophy and Progression of the Disease.
11:50 - 12:25 PM	Polling, Questions and Large Group Discussion on Symptoms and Impact of Peroxisomal Disorders.
12:25 - 12:30 PM	Panel 1 Closing Remarks
12:30 - 2:30 PM	Lunch
2:30 - 2:35 PM	Welcome Back
2:35 - 3:50 PM	Panel #2: Current Approach to Treatments
2:35 - 2:45 PM	Current Approach to Treatments Panel
2:45 - 3:35 PM	Polling and Large-Group Facilitated Discussion for Current Approach to Treatments
3:35 - 3:50 PM	Polling and Large-Group Facilitated Discussion on Meaningful Improvement in Peroxisomal Disorders
3:50 - 3:55 PM	Break
3:55 - 5:00 PM	Panel #3: Clinical Trial Readiness Panel
3:55 - 4:05 PM	Clinical Outcomes and Barriers to Clinical Trials Panel
4:05 - 4:55 PM	Polling and Large-Group Facilitated Discussion on Clinical Trial Readiness
4:55 - 5:00 PM	Closing Remarks