

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
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
3:55 - 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

EL-PFDD OVERVIEW

SPEAKERS

Sarah Fuchs, MSCI MD
William Rizzo, MD
Mousumi Bose, PhD
Melissa Bryce
Chris Ostertag, PhD

PANELISTS

Angela Feist
Vicky Maag
Daniela Vargas Ramirez
Katie Sacra
Joleen Burdick
Erasmus Torres
Melinda Torres
Andy Betzer
Todd Chapman
Jasmine Buchanan
Patty Delsorbo
Suzanne Collins
Tammy LaMaire
Sarah Heath
Kelly Dauer-Hubschmitt
Jennifer Knox

**SHARE YOUR
EXPERIENCE DURING
THE EL-PFDD MEETING**



WHAT IS THE EXTERNALLY-LED PATIENT-FOCUSED DRUG DEVELOPMENT MEETING?

The centerpiece of the 2024 GFPD Family and Scientific Conference is an Externally - Led Patient - Focused Drug Development (EL-PFDD) meeting. Since 2015, the Food and Drug Administration (FDA) has given 176 patient advocacy groups an opportunity to plan and host an EL-PFDD meeting. The GFPD is one of only seven groups hosting an EL-PFDD meeting in 2024.

Peroxisomal disorders are an ideal candidate for an EL-PFDD meeting because they are chronic and affect the functioning and activities of daily living. The majority of an EL-PFDD meeting is dedicated to listening to patient and caregiver perspectives on the condition, especially the symptoms and daily impacts of the disease, as well as the treatments that would most improve their quality of life. Decision-makers at the FDA and other government agencies, medical product developers, academic researchers, clinicians, and healthcare professionals will listen to and learn from experts in the field- the patients and their caregivers.
