VIRTUAL PMD SYMPOSIUM

Saturday, July 25, 2020 • 10 AM

Agenda



Children's Hospital of Philadelphia®

PMD FOUNDATION
PELIZAEUS-MERZBACHER DISEASE

GLOBAL
LEUKODYSTROPHY
INITIATIVE

*times are approximate

PART 1: WELCOME

10:00AM Overview and Introduction to PMD Foundation
Dave Manley

PART 2: POTENTIAL TREATMENTS IN ANIMAL MODELS

CELL TRANSPLANTATION APPROACHES

10:15AM Oligodendrocyte Progenitor Cell Transplantation
Joana Osório, MD

10:30AM Neural Stem Cell Transplantation
Julia Edgar, BSc(Hons), PhD, FHEA

GENE SUPPRESSION APPROACHES

10:45AM Correction of PLP1 Splicing with Antisense Oligonucleotides
Grace Hobson, PhD

11:00AM PLP1 Suppression with Antisense Oligonucleotides
Paul Tesar, PhD

11:15AM PLP1 Suppression with microRNA
Ken Inoue, MD, PhD

11:30AM Ketogenic Diet
Gesine Saher, PhD



Coffee Break – 11:45AM - 12:00PM

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PART 3: CLINICAL TRIALS

12:00PM Clinical Trial Overview
What does it take to get a drug to market? What does it mean to repurpose a drug?
Michael Levy, MD, PhD

12:10PM StemCells Inc Clinical Trial & 5 Year Follow-Up
David Rowitch, MD, PhD

12:20PM Clinical Trial of Iron Chelator
Nicole Wolf, MD, PhD

12:30PM MRI Outcomes and Tools in Clinical Trials
Inga Harting, MD

Coffee Break – 12:30PM - 12:45PM

PART 4: LIVING WITH PMD

12:45PM Message from Families
Questions to be previewed on screen and then addressed by up to three families who will have pre-recorded their responses.

1:15PM Live Polling
Participants will be invited to answer a number of basic questions regarding their own experiences directly via the livestream.

1:30PM Natural History Studies
What is the Global Leukodystrophy Initiative Clinical Trials Network? What are they, why are they important and how can you participate? What has been learned about PMD thus far? How might this help moving forward?
Adeline Vanderver, MD

1:45PM Introduction to Ionis Disease Burden Survey
Patrick Cauntay

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PART 5: QUESTIONS & ANSWERS FOR FAMILIES

2:00PM **Live Q&A Session for Families (30 Minutes)**
Panel of all investigators available to address questions from families.

THANKS FOR JOINING US!

