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
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*
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!