

# 2017 GLOBAL LEUKODYSTROPHY INITIATIVE CONFERENCE

## *Clinical Trial Readiness in the Leukodystrophies*

Wednesday, Nov. 8, through Friday, Nov. 10

The Global Leukodystrophy Initiative (GLIA) organizing committee is excited to announce this year's annual scientific conference, which will focus on clinical trial readiness and expanded newborn screening in the leukodystrophies. The three-day event will be hosted by the Leukodystrophy Center at the Children's Hospital of Philadelphia (CHOP).

Partners from clinical, academic and industry sectors will provide updates on existing clinical trials and disease-specific natural history studies. The overall goal of the event is to outline feasible pathways for the development of the next generation of clinical trials. We have also partnered with patient advocacy organizations and legislative experts to organize a full-day breakout session dedicated to expanded newborn screening in the leukodystrophies.

We would be thrilled if you would join us for this event.

### **Location**

Conference Room 120A/B, First Floor • Roberts Center for Pediatric Research  
Children's Hospital of Philadelphia • 2716 South St. • Philadelphia, PA 19146

### **Travel and Hotel Arrangements**

All guests will be responsible for making their own travel arrangements and hotel reservations. The conference organizers will reimburse travel expenses up to a set limit **for confirmed speakers only**. Please contact Omar Sherbini for more details and to arrange accommodations. All other guests are responsible for their own accommodations.

### **Parking**

Guest parking is available free of charge at the Roberts Center for Pediatric Research (RCPR). Parking passes can be obtained at the event.

### **Administrative Contact for Meeting**

Omar Sherbini, Research Assistant II, Division of Neurology  
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# AGENDA

Day 1      Wednesday, Nov. 8

**8:30-9 a.m.      Breakfast and Conversation**

9-9:25 a.m.      **Brief Introduction**  
*Adeline Vanderver, MD*  
*Children's Hospital of Philadelphia*

**Session 1: Surrogate Outcomes and Biomarkers**

9:30-9:55 a.m.      **Genomic Biomarkers for Leukodystrophies**  
*Kendrick Goss, PhD*  
*bluebird bio, Inc.*

10-10:25 a.m.      **Myelin Water Imaging**  
*Sean Deoni, PhD*  
*Children's Hospital of Colorado*

10:30-10:55 a.m.      **CSF Biomarkers**  
*Troy Lund, MD, PhD*  
*University of Minnesota*

11-11:25 a.m.      **Clinical Outcomes Assessment**  
*Michelle Campbell, PhD*  
*U.S. Food and Drug Administration*

11:30-11:55 a.m.      **MRI as a Surrogate for Clinical Endpoint**  
*Brenda Banwell, MD*  
*Children's Hospital of Philadelphia*

**Noon-1 p.m.      Lunch**

**Session 2 and 3: Functional Outcome Measures and Natural History Studies**

1-1:25 p.m.      **Outcomes Measures for Dystonias and Movement Disorders**  
*Liana Rosenthal, MD*  
*Johns Hopkins University*

1:30-1:55 p.m.      **Natural History Outcomes for Alexander Disease (AxD)**  
*Amy Waldman, MD*  
*Children's Hospital of Philadelphia*

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Day 1

Wednesday, Nov. 8 *continued*

- 2-2:25 p.m. **Natural History Outcomes for Metachromatic Leukodystrophy (MLD)**  
*Nicole Wolf, MD, PhD*  
*Vrije Universiteit, Amsterdam, The Netherlands*
- 2:30-2:55 p.m. **Natural History Outcomes for POLR3-related (4H) Leukodystrophy**  
*Genevieve Bernard, MD, MSc*  
*McGill University*
- 3 – 3:25 p.m. Coffee and Conversation**
- 3:30-3:55 p.m. **Natural History Outcomes for Krabbe Disease**  
*Maria Escolar, MD, MS*  
*University of Pittsburgh*
- 4-4:25 p.m. **Canavan Disease**  
*Heather Lau, MD*  
*New York University*
- 4:30-4:55 p.m. **Natural History Outcomes for Adrenoleukodystrophy (ALD)**  
*Florian Eichler, MD*  
*Massachusetts General Hospital*
- 5-5:25 p.m. **FDA Perspective on Natural History Outcomes**  
*Rachel Witten, MD*  
*U.S. Food and Drug Administration*
- 5:30-6:30 p.m. **Workshop: Building the Ideal Natural History Study**
- 7 p.m.-TBD Networking Event at Butcher Bar (Hors d'Oeuvres provided)**

Day 2 Thursday, Nov. 9

**8:30-9 a.m. Breakfast**

**Session 4: Preclinical Data for Future Leukodystrophy Therapies**

9-9:25 a.m. **Krabbe Dog Model**  
*Charles Vite, DVM, PhD – University of Pennsylvania*  
*Allison Bradbury, PhD – University of Pennsylvania*

9:30-9:55 a.m. **Nanotherapeutics in ALD**  
*Ali Fatemi, MD, MBA*  
*Kennedy Krieger Institute*

10-10:25 a.m. **iPSC-derived Oligodendrocyte**  
*Paul Tesar, PhD*  
*Case Western Reserve University*

**10:25-10:55 a.m. Coffee and Conversation**

11-11:25 a.m. **Antisense Oligonucleotides in Alexander Disease**  
*Albee Messing, VMD, PhD – University of Wisconsin – Madison*  
*Amy Waldman, MD – Children’s Hospital of Philadelphia*

**11:30-1:30 p.m. Lunch Presentation: Leukodystrophy Family Forum**

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Day 2

Thursday, Nov. 9 *continued*

**Session 5: Clinical Trial Design in the Leukodystrophies and Other Rare Diseases**

*Moderators: Raphael Schiffmann, MD – Baylor Scott & White Research Institute  
Florian Eichler, MD – Massachusetts General Hospital*

1-1:25 p.m.

**Metachromatic Leukodystrophy (MLD) Gene Therapy Trials**

*Alessandra Biffi, MD  
Boston Children's Hospital*

1:30-1:55 p.m.

**Trials in Fabry and Neuronopathic Gaucher Diseases**

*Raphael Schiffmann, MD  
Baylor Scott & White Research Institute*

2-2:25 p.m.

**Trials in Friedreich's Ataxia**

*David Lynch, MD, PhD  
Children's Hospital of Philadelphia*

2:30-2:55 p.m.

**Trials in Niemann-Pick Disease Type C**

*Marc Patterson, MD  
Mayo Clinic*

3-3:25 p.m.

**Stem Cell Therapy for Remyelination**

*Piotr Walczak, MD, PhD  
Johns Hopkins University*

**3:30-3:55 p.m. Coffee and Conversation**

4-4:25 p.m.

**Giant Axonal Neuropathy (GAN)**

*Diana Bharucha-Goebel, MD  
National Institutes of Health (NINDS)*

4:30-4:55 p.m.

**Adrenomyeloneuropathy (AMN) Trials**

*Uwe Meya, MD, PhD  
Minorxy Therapeutics*

5-5:30 p.m.

**Metachromatic Leukodystrophy (MLD) Enzyme Replacement Therapy Trials**

*Margaret Wasilewski, MD  
Shire Pharmaceuticals*

6 p.m.-TBD

**Working Dinner: MLD Newborn Screening**

Day 3

Friday, Nov. 10

**Rapid Diagnosis: Newborn Screening and Genomics**

**8:30-9 a.m. Breakfast**

**Session 1: NBS Status by Disease**

9-9:25 a.m. **Krabbe and ALD Newborn Screening: A State Lab's Perspective**

*Mark Morrissey, PhD*

*New York Department of Public Health*

9:30-9:55 a.m. **CTX Newborn Screening**

*Andrea DeBarber, PhD*

*Oregon Health and Science University*

**10-10:25 a.m. Coffee and Conversation**

10:30-10:55 a.m. **ALD Newborn Screening Implementation: Lessons from Washington State**

*Brad Zakes, MS, MBA*

*Cerevast Medical, Inc.*

11-11:30 a.m. **MLD NBS Assay Development**

*Michael Gelb, PhD*

*University of Washington*

11:30 a.m.-

Noon

**Newborn Screening Test Performance**

*Silvia Tortorelli, MD, PhD*

*Mayo Clinic*

**Noon-1 p.m. Lunch**

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Day 3 Friday, Nov. 10 *continued*

**Session 2: Newborn Screening Implementation and Policy**

- 1-1:25 p.m. **Evidence-based Review Process**  
*Alex Kemper, MD, MPH, MS*  
*Nationwide Children's Hospital*
- 1:30-1:55 p.m. **NBS Public Health and Clinical Research Perspectives**  
*Scott Shone, PhD*  
*RTI Center for Newborn Screening, Ethics and Disability Studies*
- 2-2:25 p.m. **Implementation: Challenges and Solutions (Advocacy Perspective)**  
*Elisa Seeger*  
*Aidan Jack Seeger Foundation*
- 2:30-2:55 p.m. **State and Federal Roadmaps to Implementation**  
*Elisa Seeger – Aidan Jack Seeger Foundation*  
*Dean Suhr – MLD Foundation*
- 3-3:25 p.m. Coffee and Conversation**
- Special Roundtable Session**  
*Moderator: Dean Suhr – MLD Foundation*
- 3:30-4:30 p.m. • **Capability and Ethics: Are They at Odds with Each Other?**  
• **Screening in the Absence of Disease-altering Therapies**  
• **Heterogeneous, Slowly Emerging and Later Onset Pathologies**  
• **Cost vs. Benefit**
- 4:30-5 p.m. **Closing Remarks by Organizing Committee**