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
Abramson Research Center • 516H • 3615 Civic Center Blvd.
Philadelphia, PA 19104 • 215-590-3068 • sherbinio@email.chop.edu

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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)**

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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 Insitutes 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**

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