

# NEWSLETTER



## EDUCATE • COLLABORATE • ADVOCATE

The **2024 ULF Scientific Symposium and Family Conference** is quickly approaching! We are thrilled to offer this preview, and hope you will join us in-person in Itasca, Illinois, USA in June, or virtually through Zoom!

**REGISTER TODAY**

[WWW.ULF.ORG](http://WWW.ULF.ORG)

**SCIENTIFIC SYMPOSIUM • JUNE 27**

**FAMILY CONFERENCE • JUNE 28/29**

While at the conference, be sure to visit the **Dr. Paul Watkins Excellence in Research Poster Presentation**. Researchers, academics, and industry professionals will present new and innovative leukodystrophy research. Posters will be available for viewing throughout the conference.

# A LETTER FROM THE PRESIDENT OF THE BOARD

**Greetings.** I hope this newsletter finds you all well and as eager to renew acquaintances as I am at our Annual Conference from June 27-29th at the Eaglewood Resort in Itasca, Illinois.

Organizing the Conference requires a significant amount of time from our staff and board members, but the outcome is always worth the effort. I strongly believe that this is the leading conference on leukodystrophy-related topics. The Conference offers a unique opportunity to bring together people affected by leukodystrophies, their families, and many passionate patient advocacy groups, clinicians, and researchers who dedicate their lives to providing the best care for those with leukodystrophies.

This year's Conference is exceptionally affordable thanks to the generous support of our industry partners. For less than \$150 per day, you can enjoy a comfortable room at a resort-style hotel with a large indoor pool, a variety of exercise equipment, a lively pub, and three buffet-style meals daily that cater to dietary restrictions. However, while Eaglewood is a fantastic venue, the main reason we gather here is to learn from and collaborate with the world's most respected leukodystrophy experts and the advocacy groups that support individuals affected by this condition.

With that in mind, this year's schedule is packed with informative and empowering sessions to assist those of us who live with this condition daily. Some of the highlights include discussions about the most recent updates on ongoing research, advancements in gene therapy, speech therapy, and physical therapy, an explanation of the complexities of genetic inheritance, and an overview of the different types of treatments that are currently available for the various types of leukodystrophies. Furthermore, there will be several sessions that focus on the psychological effects that accompany these diagnoses. You will have the chance to participate in or listen to discussions about dealing with grief, how to communicate with unaffected siblings, how to prevent caregiver burnout and frank conversations about the mental health challenges that individuals affected by these conditions and their loved ones face.

There will also be half-day mini symposiums that are dedicated to some of the specific subtypes such as Vanishing White Matter Disease, Cerebrotendinous Xanthomatosis (CTX), Adrenomyeloneuropathy/Adrenoleukodystrophy (AMN/ALD), Metachromatic Leukodystrophy (MLD), Adult-Onset Leukoencephalopathy with Axonal Spheroids and Pigmented Glia (ALSP), Krabbe Disease, Megalencephalic Leukoencephalopathy with Subcortical Cysts (MLC), and Cadasil. These sessions will be led by leading researchers who specialize in the specific disorder, along with a number of our partners in advocacy who assist their members on a daily basis. The Conference will also provide ample opportunity for you to meet and talk with these experts one-on-one over a cup of coffee or share experiences with others who understand the challenges and joys that come with the path we are on.

In conclusion, I strongly encourage you to attend this remarkable Conference, which is designed for your benefit. It was and still is, a life-changing experience for me and my family, and I believe it can offer you the same.



*Ron, Marla, and their son Aiden*

Best wishes to all,

**RON CHAPLEAU**

*ULF Board President*

# CONFERENCE SPONSORS:



## TYPE-SPECIFIC CONTENT & CONTENT PARTNERS 2024:



MLC content presented by  
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MLD content presented by  
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CTX content presented by  
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Krabbe content presented by  
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CADASIL content presented by  
**Cure CADASIL**



VWM content presented by  
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### Other type-specific sessions presented by the ULF:

- Alexander Disease
  - Pelizaeus Merzbacher Disease (PMD)
  - Adult-Onset Leukoencephalopathy with Axonal Spheroids and Pigmented Glia (ALSP)
  - Adrenoleukodystrophy (ALD)
  - Adrenomyeloneuropathy (AMN)
  - Labrune Syndrome: Leukoencephalopathy, Calcifications, and Cysts (LCC)
  - L-2 Hydroxyglutaric Aciduria
- and more!**

# 2024 SCIENTIFIC SYMPOSIUM

THURSDAY, JUNE 27 - 8AM-4:30PM CENTRAL - ITASCA, ILLINOIS, USA

**Our Scientific Symposium** is open to the academic and professional level audience: doctors, researchers, allied health professionals, industry representatives, nonprofit partners, and advocacy organizations. For our affected families, we will provide summaries of the sessions that are geared towards the family audience on Saturday, June 29.

SESSION TOPIC	SPEAKER
Adult Polyglucosan Body Disease: 2024 Update	Dr. Alexander Lossos, Hadassah-Hebrew University Medical Center
Advancements for Gene Therapy Leukodystrophies	Dr. Allison Bradbury, Nationwide Children's Hospital
Advances in MSD Diagnosis, Phenotype and Treatment	Dr. Laura Adang, Children's Hospital of Philadelphia
Can We Achieve Meaningful Gene Correction in the Leukodystrophies?	Dr. Florian Eichler, Massachusetts General Hospital
Comprehensive Evaluation of Genotype-Phenotype Relationships in Pol-III Related Disorders	Mackenzie Michell-Robinson, PhD Candidate, McGill University
Functional Characterization of the IFIH1 Mutations in Aicardi-Goutieres Syndrome: Cytosolic RNA Sensors and Novel Molecular Regulators	Erika Maghraby, PhD Candidate, University Children's Hospital V. Buzzi
Latest News on Leukodystrophy Research from Amsterdam	Dr. Marjo S. van der Knaap, Amsterdam University Medical Center
Leukodystrophies: Much More than Just Diseases of Myelin	Dr. Marianna Bugiani, Amsterdam University Medical Center
Leukoencephalopathy with Calcifications and Cysts: Natural History and Clinical Management Considerations	Dr. Jamie Fraser, Children's National Hospital
Macrophage Transplantation Rescues RNASET2-Deficient Leukodystrophy by Replacing Deficient Microglia in a Zebrafish Model	Holly A. Rutherford, PhD Candidate, Sheffield University
Natural History and Imaging Biomarkers of Early-Stage Cerebral Adrenoleukodystrophy	Dr. Erik Mallack, Kennedy Krieger Institute
NIH Research and Neurogenetic Disorders	Dr. Walter Koroshetz, National Institutes of Health
Real-World Data Supporting Use of CDCA in Pregnancy in CTX	Dr. Tzipora Falik-Zaccai, Galilee Medical Center, Ban-Ilan University



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# 2024 FAMILY CONFERENCE

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FRI-SAT, JUNE 28-29 - 8:30AM-5PM CENTRAL - ITASCA, ILLINOIS, USA

## FAMILY CONFERENCE GENERAL INTEREST SESSIONS:

These sessions will be streamed live to our virtual attendees and recorded. We anticipate the videos being posted to YouTube in early August. Topics and speakers are subject to change.

### **Advancements in Gene Therapy**

presented by Dr. Florian Eichler, Massachusetts General Hospital

Gene therapy continues to make strides as a treatment option for some leukodystrophies!

### **Anticipatory Grief**

presented by Nicollette Vasquez, Lightways Joliet

A gentle session that addresses how to manage the grieving process before it even truly begins.

### **Bioethics: Understanding the Importance of Informed Consent**

presented by Melody Kisor, Cure LBSL

Informed consent is a huge concern for everyone, but especially those participating in treatment including clinical trials and experimental medicines.

### **Choosing Medical Equipment**

presented by Dr. Kathy Zackowski and Jennifer Keller, Kennedy Krieger Institute

Physical therapists address the things to consider when you're looking to acquire medical equipment.

### **Exploring the Benefits of Hippotherapy or Therapeutic Horseback Riding**

presented by Christine Hanebury, Sebastian Riding Associates

Therapeutic horseback riding has tremendous impacts on people affected by all sorts of disorders and diseases. Learn more about how hippotherapy could be right for you or your loved one with leukodystrophy.

### **Forecasting Interventions for Pediatric Patients**

presented by Dr. Stephanie Keller, Emory University

A brief introduction for parents and caregivers into medical interventions that could be options for pediatric patients, such as feeding tubes, respiratory assistance, and more.



## **Introducing: the ULF's Ambassador Program**

presented by Keely Mata and the ULF Pilot Ambassadors

The ULF Ambassador Program's official launch! Learn more about how to leverage the ULF Ambassadors as an affected family to access resources and information, and how to volunteer!

## **Leukodystrophy 101**

presented by Dr. Ali Fatemi, Johns Hopkins Medical Center and Kennedy Krieger Institute

A brief introduction into what it means to have a leukodystrophy. This session is intended for newly diagnosed families or people seeking more information about leukodystrophies as a family of diseases.

## **Making the Most of Your Wish: a Make-A-Wish Alumni Panel**

presented by Dina Thachet, Make-A-Wish Illinois and Wish Alumni

The possibilities are endless with Make-A-Wish – but how do you make the most out of your wish while also being cognizant of your affected person's abilities? Leukodystrophy Wish Alumnae share their stories.

## **Managing Caregiver Burnout**

presented by Marla Chapleau, MLC Mom Caregiver and Alliance MLC

The first step is recognizing burnout, and knowing you are not alone. Taking steps to address burnout and find strategies that are right for you protects you as a caregiver, and your affected person.

## **Mental Health for Affected Adults: Processing a New Diagnosis**

presented by Sarah Stoney, Children's Hospital of Philadelphia, and Soo Shim, Lurie Children's Hospital

Insights into protecting your mental health as an affected adult with a new leukodystrophy diagnosis while also focusing on physical health concerns.



## **Mental Health for Parents of Affected Children: Processing a New Diagnosis**

presented by Sarah Stoney, Children's Hospital of Philadelphia, and Soo Shim, Lurie Children's Hospital

A child with a diagnosis presents many obstacles, and mental health of the parents and caregivers are an important aspect of the overall quality of care and treatment of the child.

## **Newborn Screening Crash Course – Get Involved in Your State!**

presented by Lesa Brackbill, Leukodystrophy Newborn Screening Action Network

Newborn Screening for leukodystrophies have made huge strides in the past few years! Keep that momentum going in your state with the help of the Leukodystrophy Newborn Screening Action Network.

## **Physical Therapy for Adults**

presented by Dr. Kathy Zackowski and Jennifer Keller, Kennedy Krieger Institute

Physical therapists offer tips to affected adults.

## **Scientific Symposium Session Summaries**

presented by Dr. Geneviève Bernard, McGill University and Chair of the ULF Medical and Scientific Advisory Board

Summaries of the Scientific sessions that are geared towards a non-medical audience.





## Speech Therapy Topics

presented by Tempe Thomas, Vox Chicago

A speech therapist offers guidance on speech therapy for affected individuals, open to all.

## Treatment Modalities for Leukodystrophies

presented by Dr. Ali Fatemi, Johns Hopkins and Kennedy Krieger Institute

How are leukodystrophies treated? How does the type of leukodystrophy dictate the mode of treatment that's appropriate? Learn more about the types of treatment strategies available to the leukodystrophy community.

## The Unaffected Siblings: A Session for Parents

presented by Sarah Stoney, Children's Hospital of Philadelphia,  
Loren Mirsky-Piatkin, Breakthrough Coaching Center,  
and Alex and Liam Chapleau, MLC Siblings

A presentation and discussion panel for parents of affected and unaffected children, on how to recognize signs of crisis and gain insight into how the unaffected sibling(s) are themselves affected by leukodystrophies.

## Unlocking Your Family History and Decoding Genetic Inheritance

presented by Rachel Hickey, Lurie Children's Hospital

Chromosome, autosome, dominant, recessive – what do these words mean, and why do they dictate if a person is going to be affected by leukodystrophy? Explore the world of genetics and familial history of disease.

## Updates from our Industry Partners

presented by our sponsors

Our sponsors and industry partners share the latest information on their efforts to bring new therapies to the leukodystrophy community.

## Utilizing Palliative Care and Hospice for Quality of Life

presented by Dr. Erin Flanagan, Lightways Joliet

What is the difference between palliative care and hospice care? And how can either (or both) be used to ensure quality of life for a person affected by leukodystrophy?

## What to Expect? Anticipatory Guidance for Parents of Affected Children

presented by Lindsey Topping Schuetz, Courageous Parents Network

A brief look at multiple topics that a parent might encounter as they care for their affected child throughout the journey, and gentle guidance on tackling challenges at each stage of the neurological disease progression.

## Zebrafish, Mice, and More: Research Strategies for Leukodystrophies

presented by Dr. Josh Bonkowsky, Utah Health

Ever wondered about all the different ways that researchers go about their projects? Learn more about research strategies from one of the ULF's Medical and Scientific Advisory Board members!





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