

NEWSLETTER



A RENEWED VISION FOR THE ANNUAL ULF CONFERENCE

The ULF's **Annual Scientific Symposium and Family Conference** is quickly approaching, and this year is shaping up to be one of the most exciting events ever for the ULF community. The ULF's mission is to empower the entire leukodystrophy community – affected people and families, caregivers, support people, medical professionals, researchers, industry partners, and our fellow patient advocacy groups. In the past couple of years, the ULF has collaborated with type-specific organizations to bring content to the Family Conference that is focused on the topics that the type-specific groups need. This year, we are thrilled to be partnering with ten organizations to bring **type-specific content** to our meeting!

On June 23 and 24, we welcome the AGS Advocacy Association, Alliance MLC, CTX Alliance, Cure CADASIL, Cure LBSL, Cure MLD, Foundation to Fight H-ABC, Global DARE Foundation (Refsum), Sister's Hope (ALSP), and VWM Families Foundation as our Content Partners! Each of these organizations are hosting a half-day session dedicated to information that is specific to their respective types of leukodystrophy. The ULF is working diligently to provide content that is beneficial to all leukodystrophy families, regardless of their type-diagnosis. This new approach to our agenda will allow us to ensure that type-specific content is being expertly covered, while still providing content that aims to improve the quality of life for everyone on the leukodystrophy journey. We hope our collaboration brings a renewed sense of togetherness as we move into the future.

This year's meeting is in-person, but we will have virtual access to all sessions excluding support groups. Streamed sessions will be recorded and posted to our YouTube channel in July. Please keep in mind that our agendas are subject to change, so the best way to stay up to date is to check out our website! This newsletter features session highlights to give you an idea of what to expect when you join us in June.

ULF FAMILY CONFERENCE
JUNE 23 - 24, 2023
ITASCA, ILLINOIS, USA

REGISTER TODAY!
ULF.ORG/CONFERENCE

A LETTER FROM THE PRESIDENT OF THE BOARD

Hello again everyone. It's nice to open the curtains and greet sunshine and greenery once again after yet another exceptionally long winter.

Here at the ULF, we are well into what can only be termed as “our busy season”. Plans are being finalized for our annual conference in Itasca, Illinois (just outside of Chicago) at the fabulous Eaglewood and Spa running on June the 22nd through the 24th and it looks like it has the potential to be the best one ever.

A couple of years ago our board sat down and tried to think of how the ULF, an organization that represents ALL leukodystrophies, can best continue to serve our collective community. Of course, this includes our affected families and individuals, the researchers and clinicians who have made these diseases their life's work and our many partners in the patient advocacy community. One of the ways we could best accomplish our goal was to transform our annual Family Conference and Scientific Symposium into a more inclusive event. Hosting a conference is an extremely expensive proposition both in terms of money and manpower. It struck us as wasteful for each and every patient advocacy group to pour their resources into holding many individual conferences when that same money could be better spent on supporting research or families in need. It was also apparent that asking our dedicated scientific community to attend multiple conferences each year imposed a hardship on both them and their families.

As such, we reached out to many of the amazing advocacy groups and offered to partner with them in holding this conference. We are delighted that so many have accepted (AGS Advocacy Association, Alliance MLC, CTX Alliance, Cure CADASIL, Cure LBSL, Cure MLD, Foundation to Fight H-ABC, Global DARE Foundation (Refsum), Sister's Hope (ALSP), and VWM Families Foundation) and, as such, they will be running mini-conferences with their own agendas and speakers. It is this type of collaboration that will serve to drive the entire community forward at an even faster pace. It is our hope that in future years we will continue to add additional advocacy groups to the Conference as we act together for the collective betterment.

Please take a second to visit our website and see all the presentations that are relevant for just about anyone affected by a leukodystrophy. Topics covered, to name only a few, include:

-
- expert advice on overcoming communication challenges
 - advice from experienced physio and occupational therapists
 - a discussion on the differences between gene, cell, and drug therapy
 - advice on vision therapy
 - and, for the first time, professional coaching on how to share your story and the impact it can have on others including the decision makers in the government such as the FDA.
-

I am looking forward to renewing and making new acquaintances with many of you this June. Can't wait to see you all in Itasca!



Ron, Marla, and their son Aiden

RON CHAPLEAU

ULF Board President

2023 SCIENTIFIC SYMPOSIUM

THURSDAY, JUNE 22 - 8AM-4:15PM CENTRAL - ITASCA, ILLINOIS, USA

Your registration to the Scientific Symposium includes access to the Family Conference as well! Be sure to check out the separate agendas for the Family Conference days of Friday, June 23 and Saturday, June 24 at ulf.org/conference.

Agenda is subject to change. Visit ULF.org/Scientific-Symposium for more info and to register!

TIME	SESSION TOPIC	SPEAKER
8:00-8:15 am	ULF Welcome	Dr. Genevieve Bernard, ULF Board and Staff
8:15-8:45 am	News from Amsterdam	Dr. Marjo S. van der Knaap, Amsterdam UMC
8:45-9:15 am	Leukodystrophies with disturbed brain water homeostasis – new genetic insights	Dr. Rogier Min, Amsterdam UMC
9:15-9:45 am	Cerebral vasculopathy – more than just a pathological feature in Aicardi-Goutières syndrome?	Dr. Markus Hofer, University of Sydney, Australia
9:45-10:00 am	BREAK	
10:00-10:30 am	CADASIL-centered modeling of vascular leukoencephalopathy	Dr. Fanny Elahi, Mount Sinai – New York
10:30-11:00 am	Retrospective natural history study in PMD: approach and findings	Dr. Anjana S. Sevagamoorthy, Children’s Hospital of Philadelphia
11:00-11:30 am	POLR3-related (4H) leukodystrophy: a research update on disease modeling	Julia Macintosh, McGill University
11:30 am-12:00 pm	Newborn screening for CTX: considerations towards implementation	Dr. Hidde Huidekoper, Erasmus MC
12:00-1:00 pm	LUNCH	
1:00-1:30 pm	Structural insight into VLCFA transport by human Adrenoleukodystrophy Protein/ABCD1	Dr. Amer Alam, University of Minnesota
1:30-2:00 pm	Morbidities and mortality in adult men with AMN	Dr. Joshua Bonkowsky, University of Utah
2:00-2:30 pm	The mouse peroxisome research resource	Dr. Joseph Hacia, University of Southern California
2:30-2:45 pm	BREAK	
2:45-3:15 pm	Combined transcriptome-translatome analysis of eIF2B-mutant astrocytes along cytokines-mediated activation discovers the reason for their incompatibility	Dr. Orna Elroy-Stein, Tel Aviv University
3:15-3:45 pm	Biomarkers and outcome measures in AxD	Dr. Amy Waldman, Children’s Hospital of Philadelphia
3:45-4:15 pm	Antisense oligonucleotide therapy in leukodystrophies	Dr. Manouchehr Amanat, Kennedy Krieger Institute

2023 FAMILY CONFERENCE

FRI-SAT, JUNE 23-24 - 9AM-5:30PM CENTRAL - ITASCA, ILLINOIS, USA

We hope you will join us for dinner on Thursday, June 22 as we kick off the weekend!

This agenda is subject to change. Visit [ULF.org/Conference](https://ulf.org/conference) for more info and to register!

- Welcome!
ULF Board of Directors and Staff
- Leukodystrophy 101
Dr. Jennifer Rubin, Lurie Childrens Hospital
- MLD, Cure MLD
- MLC, Alliance MLC
- Refsum, Global DARE Foundation
- LBSL, Cure LBSL
- Making Memories with Your Loved Ones
Jamie & Fahr Juneja, Eva's Butterfly Wishes
- Sharing Your Story Presentation
*Bill Berry, Berry & Company
Public Relations*
- Forming Your Medical Team
*Dr. Stephanie Keller,
Children's Hospital of Atlanta*
- Genetic Counseling and Family Planning
Julie Cohen, Kennedy Krieger Institute
- One-on-One Storytelling Coaching
*Bill Berry, Berry & Company
Public Relations*
- Modalities of Leukodystrophy Treatment
- Caregiving the Caregiver
*Marcie Flores & Donna Skwirut,
ULF Board of Directors*
- Communication Aids
*Beth Christiansen & Sayard Bass,
Speech Pathologists*
- Undiagnosed & Ultra-Rare Leukodystrophies
- VWM, VWM Families Foundation
- AGS, AGSAA
- H-ABC, Foundation to Fight H-ABC
- Newborn Screening
Lesa Brackbill, Luekodystrophy NBS Action
- Adaptive Toys
Shannon Reed, ULF Board of Directors
- The Unaffected Siblings: Parents Discussion
Liam and Alex Chapleau, MLC Siblings
- Palliative and Hospice Care
Dr. Terese Vente, Lurie Children's Hospital
- Physical Therapy Topics
*Dr. Kathy Zackowski and Jennifer Keller,
Kennedy Krieger*
- Scientific Symposium Session Summaries
*Dr. Genevieve Bernard, ULF MSAB Chair,
McGill University*
- Disease Research: A Complex Journey
*Dr. Paul Watkins, Kennedy Krieger Institute,
and Dr. Joe Hacia, USC*
- ALD/AMN, ULF
- CADASIL, Cure CADASIL
- Alexander Disease, ULF
- CPR Training
Jon Ritter, NREMT-P
- Travel Planning with Leukodystrophies
Meredith Maki, Travel Agent
- Insights from Moms Panel
*Marilyn Schmiedel, Margaret McIntyre,
Marcie Flores, Jenny Arnold*
- Brain and Tissue Banking
Anne Rugari, Partners for Krabbe Research
- Accessible Home Design
- Insights from Dads Panel
Kurt Triptow, David Flores
- Speech Therapy
*Tempe Thomas, Manisha Jain,
Speech Pathologists*
- Service Animals
*Tom and Debra Johnson,
Service Dog Owners, CTX*
- Support Group: Latinx
Marcie Flores, ULF Board of Directors
- Navigating Emergency Room and
Hospital Visits
Jenny Arnold, VWM Mom
- CTX, CTX Alliance
- 4H Hangout, Yaya Foundation for 4H
- ALSP, Sister's Hope Foundation
- The Unaffected Siblings: Siblings Discussion
Liam and Alex Chapleau, MLC Siblings
- Restorative Yoga Workshop
Ilyce Randell, Yoga Instructor
- Support Group: Parent Caregivers
LuAnn Swartzlander
- Forward Movement in the UK
*Dr. Noemie Hamilton, Leuko Labs,
and Karen Harrison, Alex TLC*
- Parents as Teammates & Hospital
Social Workers
*Susan Agrawal and Soo Shim,
Lurie Childrens Hospital*
- Occupational Therapy
Michaja Johnson, Occupational Therapist
- Support Group: Affected Adults
JoAnn Locy, AMN
- Gene Therapy 101
Rachel Logan, Genetic Counselor
- Transitioning from Pediatric to Adult Care
*Angela Berger and Dr. Parag Shah,
Lurie Children's Hospital*
- Vision Therapy
Morgan Hendon
- Support Group: Spouses
Roger Locy

SUPPORT
GROUPS

NETWORKING
OPPORTUNITIES

FAMILY FUN &
ENTERTAINMENT

FAMILY CONFERENCE

SESSION HIGHLIGHTS:



Forming Your Medical Team

Presented by Dr. Stephanie Keller, Emory and Children's Hospital of Atlanta

Facing a new leukodystrophy diagnosis can be isolating and terrifying. How do you go about ensuring you or your loved one's medical team is complete? Dr. Keller talks about how to build the team responsible for caring for you or your affected loved one.



Navigating Emergency Room and Hospital Visits

Presented by Jenny Arnold, VWM Mom

Going to the Emergency Room is stressful enough – prepare yourself and/or your affected loved one for potential trips and resulting hospital stays by compiling the information that you'll need to crash-course educate medical professionals who might not be familiar with leukodystrophy.



CPR Training

Presented by Jon Ritter, NREMT-P CTX

The ULF is offering a class that will focus on how to perform CPR on infants, children, and adults, in case of emergencies. This three and a half hour session will need to be attended in its entirety, but will be recorded for viewing later. This skill is a life-saver, not only for our loved ones with leukodystrophy!



Service Animals

Presented by Debra and Tom Johnson, CTX

Service animals can be a wonderful resource for people with leukodystrophies! Debra and Tom talk about their experience with their service dog, Storm.



FAMILY CONFERENCE

TYPE-SPECIFIC SESSIONS:

Welcome to the 2023 Content Partners!



AGSAA

Aicardi-Goutieres Syndrome (AGS)
Friday, June 23, 2-5:30 pm



Alliance MLC

Megalencephalic Leukodystrophy with Subcortical Cysts (MLC)
Friday, June 23, 9 am-12:30 pm



CTX Alliance

Cerebrotendinous Xanthomatosis (CTX)
Saturday, June 24, 2-5:30 pm



Cure CADASIL

Cerebral Autosomal Dominant Arteriopathy with Subcortical Infarcts and Leukoencephalopathy (CADASIL)
Saturday, June 24, 9 am-12:30 pm



Cure LBSL

Leukoencephalopathy with Brain Stem and Spinal Cord Involvement and Lactate Elevation (LBSL)
Friday, June 23, 9 am-12:30 pm



Cure MLD

Metachromatic Leukodystrophy
Friday, June 23, 9 am-12:30 pm



Foundation to Fight H-ABC

Hypomyelination with Atrophy of the Basal Ganglia and Cerebellum (H-ABC)
Friday, June 23, 2-5:30 pm



Global DARE Foundation

Refsum Disease
Friday, June 23, 9 am-12:30 pm



Sister's Hope Foundation

Adult-Onset Leukoencephalopathy with Axonal Spheroids and Pigmented Glia (ALSP)
Saturday, June 24, 2-5:30 pm



VWM Families Foundation

Vanishing White Matter (VWM)
Friday, June 23, 2-5:30 pm

Other type-specific sessions to include:

- Adrenoleukodystrophy (ALD) and Adrenomyeloneuropathy (AMN)
- Alexander Disease (AxD)
- Hypomyelination, Hypogonadotropic, Hypogonadism and Hypodontia (4H Leukodystrophy)
- Undiagnosed or Unspecified Leukodystrophies

CONFERENCE SPONSORS:



TRAVERETM
THERAPEUTICS

IONIS

(vigil)
NEURO



VIKINGTM
THERAPEUTICS

Calico

minorityx
therapeutics

SwanBio
THERAPEUTICS

Leadiant
Biosciences

Orchard
therapeutics

bluebirdbio

FORGE
BIOLOGICS

affiniaTM
THERAPEUTICS

poxel

GENALI
THERAPEUTICS

Aspa
a bridgebio company

Maxie's Hope
P.O. BOX 5823
Buffalo Grove, IL 60089
maxieshope.org



224 North Second Street, Suite 2
DeKalb, IL 60115 | www.ulf.org

SUPPORT THE ULF THROUGH IN-KIND DONATIONS TO OUR SILENT AND LIVE AUCTIONS!



Always a fun time at the annual meeting are our silent and live auctions!

The auctions are a key part of fundraising to ensure that we are keeping the cost to affected families as low as possible to attend the Conference. We love handmade items, trinkets that represent your hometown (or home country!), or any sort of interesting things that might catch someone's eye!



If you're joining us in-person, bring your donation with you! Or you can mail us your item donation by June 10th to:

→ ULF
224 N. Second St., Ste. 2
DeKalb, IL 60115

Your in-kind donation
may be tax deductible!
Thank you!