

# Hunter's Hope Foundation

## 2021 Global Virtual Leukodystrophy Symposium

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**Tuesday, July 20, 2021 - 11:00am - 3:00pm EDT**

Zoom Webinar link: <https://us02web.zoom.us/j/89423226010>

Webinar ID: 894 2322 6010

### *Welcome and Overview, 11am EDT*

*Presented by: Anna Grantham*

### *State Labs Newborn Screening Updates, 11:05am - 12:55pm EDT*

State Leukodystrophy NBS Program updates -- protocols, outcomes, and upcoming plans will be presented.

*Presented by: State Newborn Screening Lab Representatives*

*Moderated by: Dr. Robert Thompson-Stone*

### *Updates in Krabbe NBS Algorithm – Dr. Dieter Matern, 11:05 – 11:15am*

*Kentucky – Dr. Sainan Wei, 11:15 – 11:25am*

*New York – Dr. Joseph Orsini, 11:25 – 11:35am*

*Missouri – Tracy Klug, 11:35 – 11:45am*

*Ohio – Dr. Margie Ream, 11:45 – 11:55am*

*Tennessee – Dr. Christine Dorley, 11:55am – 12:05pm*

*Illinois – Dr. Khaja Basheeruddin, 12:05 – 12:15pm*

*New Jersey – Caitlin Russo, 12:15 – 12:25pm*

*Indiana – Allison Forkner, 12:25 – 12:35pm*

*Pennsylvania – PJ Borandi, 12:35 – 12:45pm*

*Georgia – Dr. Patricia Hall, 12:45 – 12:55pm*

*Krabbe NBS Patient Perspective – 12:55pm*

### **Leukodystrophy NBS Updates 1:00 - 3:00pm EDT**

*Moderated by: Dr. Joseph Orsini*

### *Krabbe RUSP (Recommended Uniform Screening Panel) Nomination Update, 1:00 - 1:10pm*

This session will update family and medical attendees about the effort to nominate Krabbe Disease to the RUSP.

*Presented by: Dr. Joseph Orsini*

### *Krabbe NBS Council, 1:10 - 1:25pm*

Learn about the Council, which meets monthly to review Krabbe NBS patient cases and to continually improve standards and outcomes for Krabbe NBS.

*Presented by: Dr. Joanne Kurtzberg*

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### *Krabbe NBS Guidelines, 1:25 1:45pm*

Updated guidelines on Krabbe NBS screening protocols, patient risk classification, and short and long-term follow-up. Update on Psychosine as a biomarker for Krabbe Disease.

*Presented by: Dr. Robert Thompson-Stone*

### *IKD NBS Outcomes and Neuroimaging Standards for Krabbe Disease, 1:45 2:15pm*

*Presented by: Dr. Joanne Kurtzberg, Dr. Margie Ream, Dr. Ali Mian*

### *Update on ALD NBS, 2:15 2:30pm*

*Presented by: Dr. Eric Mallack*

### *Update on MLD NBS, 2:30 2:45pm*

An update on the NBS screening assay for MLD and outcomes of the MLD NBS pilots.

*Presented by: Dr. Michael Gelb*

### *Discussion, 2:45 3:00pm*

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**Wednesday, July 21, 2021 - 11:00am - 3:15pm EDT**

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*Welcome and Overview, 11:00 – 11:05am*

*Presented by: Anna Grantham*

*Clinical Trials and New Treatments for Leukodystrophies, 11:05 – 1:30pm*

Learn about current and upcoming clinical trials and, hopefully, new treatment options for Leukodystrophies.

*Moderated by: Dr. Joanne Kurtzberg*

*Traverse Therapeutics, 11:05 – 11:20am*

*Orchard Therapeutics, 11:20 – 11:35am*

*Ionis Pharmaceuticals, 11:35 – 11:50am*

*Forge Biologics, 11:50am – 12:05pm*

*Passage Bio, 12:05 – 12:20pm*

*Neurogene, 12:20 – 12:35pm*

*Affinia Therapeutics, 12:35 – 12:45pm*

*Gain Therapeutics, 12:45 – 12:50pm*

*Discussion*

*LCN Center Care Coordinators Transition Project, 1:05 – 1:25pm*

LCN Care Coordinators are Nurse Practitioners and RNs and are at the heart of all we do through the LCN. They will share about their Leukodystrophy Kid's Guide and Transition of Care Project.

*Presented by: Catie Becker*

*LCN Certified Centers, 1:25 – 3:15pm EDT*

Center presentations to share with other LCN Centers the services and operational procedures provided. These presentations inform medical providers and affected families about each LCN Center. *Presented by: LCN Center Directors*

*Kennedy Krieger Institute, 1:25 – 1:35pm EDT*

*Duke Children's Hospital, 1:35 – 1:45pm*

*Massachusetts General Hospital, 1:45 – 1:55pm*

*Golisano Children's Hospital, 1:55 – 2:05pm*

*Lurie's Children's Hospital, 2:05 – 2:15pm*

*Primary Children's Hospital, 2:15 – 2:25pm*

*Children's Healthcare of Atlanta, 2:25 – 2:35pm*

*Nationwide Children's Hospital, 2:35 – 2:45pm*

*UC Davis Health System, 2:45 – 2:55pm*

*Weill Cornell Medicine, 2:55 – 3:05pm*

*Oishei Children's Hospital, 3:05 – 3:15pm*

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### Thursday, July 22, 2021 - 11:00am - 3:00pm EDT

*Prayer Party, 11:00am - 11:40am EST*

Zoom Meeting link (Prayer Party): <https://us02web.zoom.us/j/84387467974>

Meeting ID: 843 8746 7974

### Medical Presentations for Leukodystrophy Families

Zoom Webinar link: <https://us02web.zoom.us/j/89423226010>

Webinar ID: 894 2322 6010

*Gene Therapy 101, 12:00 - 12:30pm EDT*

This presentation will help explain how gene therapy works and what it can potentially mean for Leukodystrophies.

*Presented by: Dr. Florian Eichler*

*Clinical Trials 101, 12:30 - 1:00pm*

This presentation will help families understand how to navigate clinical trials.

*Presented by: Dr. Joshua Bonkowsky*

*RUSP 101, 1:00 - 1:30pm*

This presentation will provide a deeper understanding of the Recommended Uniform Screening Panel - what it is, how to be added and how it will help expand NBS for Krabbe.

*Presented by: Mia Morrison*

*Leukodystrophy Care Network 1:45 - 2:15pm*

*Presented by: Catie Becker, Kim Hollandsworth and Rachel Hickey*

*Newborn Screening - Family Perspective 2:15 - 2:30pm*

*Presented by: Bruce Kirbo*

### Thursday Evening - 7:00pm EDT

Zoom Meeting link: <https://us02web.zoom.us/j/84387467974>

Meeting ID: 843 8746 7974

### Family Reunion

Each registered family in attendance will have a chance to introduce themselves and share their story.

*All are welcome to attend.*

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**Friday, July 23, 2021 - 11:00am - 3:00pm EDT**

*Prayer Party, 11:00am - 11:40am EST*

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**Meeting ID: 843 8746 7974**

### **Hunter's Hope's Family Programs and Resources**

The Hunter's Hope Family Programs are designed to meet the needs of those affected by Leukodystrophy. These sessions will inform the audience about the programs, how they are administered, how they can participate, and how we might be able to expand and enhance them.

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*Hunter's Hope Family Programs, 12:00 - 12:15pm EDT*

*Presented by: Jessica Ohlson*

#### *Wall of Fame*

Hundreds of Leukodystrophy families have contacted Hunter's Hope since we started the Foundation in 1997. The Wall of Fame is a tribute to all of their children and to everyone affected by Leukodystrophies. The Wall of Fame website will be shown with how a family can request to have their child included.

#### *Equipment and Supply Exchange*

The Equipment and Supply Exchange program is designed to help fulfill the equipment and/or supply needs of Leukodystrophy children as well as help families place equipment and supplies that they are no longer using. The program will be described with information on how families can participate.

#### *Wish Gift*

Our Wish Gift Program helps families facing extreme financial limitations attributed to the costs associated with caring for a child with Leukodystrophy. Requests are granted on a case-by-case basis and are limited to meeting needs that will enable an affected family to provide the best possible care and quality of life for their child. The program will be described with information on how families can apply.

#### *Other Family Support Programs*

There are many great resources available for families caring for a child with a Leukodystrophy. Many family advocates were involved in creating this list. The site will be reviewed with input from those attending that may be aware of additional family support programs.

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### *Outreach, 12:15 - 12:25pm EDT*

Our outreach mission is to reach families with hope and encouragement.

This session will provide families with information on the Hunter's Hope Outreach, which will include Hunter's Day of Hope & Prayer for Children, Books & Bears, and the new affected family authored Comfort Booklet.

**Presented by: Jessica Ohlson**

### *Family Resource Guide, 12:25 - 12:50pm EDT*

*(This will include a live Q&A section)*

The Family Resource Guide is a comprehensive online tool currently being developed by Hunter's Hope to help families access help quickly for a variety of needs at home and in the community. Easily learn about national guidelines, trusted not-for-profit organizations, and local contacts to find resources close to home for services, equipment, supplies, and more. A brief overview of existing supports currently in place will be discussed as well as new initiatives for the future.

**Presented by: Jessica Ohlson and Elizabeth Clay**

### *Family Fundraising, 12:50 - 1:00pm EDT*

This session will provide families with the resources they need to successfully fundraise for Hunter's Hope programs like the Family Symposium and Wish Gift.

**Presented by: Melissa Jablonski**

### *Family Breakout Sessions (Open to Affected Families Only), 1:00 - 3:00pm EDT*

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\* These sessions are reserved for family members of an affected child.

## Friday Evening - 6:00 pm EDT

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### *Virtual Family Symposium Walk*

Remember and honor loved ones -- write messages on your balloons, walk in your neighborhood, or choose a fun activity to do as a family. Share your photos on social media using #HHSymposium.

**Welcome and Kick-off: The Hunter's Hope Team**

## Friday Evening - 7:00 pm - 9:00pm EDT

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**Meeting ID: 843 8746 7974**

### *Family Hangout*

Meet new families and reconnect with old friends!