Hunter’s Hope Foundation
2020 Global Virtual Leukodystrophy Symposium

Schedule Summary

Click on sessions below to see a more detailed description.

Tuesday, July 21, 2020 (10:00am – 3:00pm)

Lunch Break (12:00pm – 12:30pm)

Medical & Science Newborn Screening
- State Labs Newborn Screening Updates (Group presentation by State)
- Leukodystrophies NBS to Treatment
- Krabbe NBS Council
- Krabbe NBS Guidelines
- EIKD NBS Case Study
- Krabbe NBS Patient Perspective Survey
- ALD NBS Guidelines
- ALD NBS Parent Guidelines
- MLD NBS Pilot and Next steps
- CTX Guidelines
- Treatment for ALD, CTX, Krabbe and MLD

Wednesday, July 22, 2020 (10:00am – 3:00pm)

Lunch Break (12:00pm – 12:30pm)

Leukodystrophy Care Network (LCN)
- LCN Coordinating Center
- LCN Certified Centers
- LCN Center Care Coordinators
- LCN Clinical Practice Guideline (CPG)
- LCN Leukodystrophy Data Registry (LDR)
- LCN Telemedicine
- LCN Resource Library
- LCN Family Perspective (Family Panel)
- LCN Open Discussion – Priorities moving forward

Wednesday Evening (7:00 p.m.)

Welcome and Family Introductions

Thursday, July 23, 2020 (11:00am – 1:00pm)

Family Perspective - Medical
- State Labs NBS Updates Family Perspective (Family Panel)
- Leukodystrophies NBS to Treatment Family Perspective (Family Panel)
- LCN Family Perspective (Family Panel)
- Hunter’s Hope Krabbe NBS Update
- Federally Recommended Uniform Screening Panel, Workgroups, and Council
- State by State – Family Advocates
Lunch (1:00pm – 2:00pm)

Thursday, July 23, 2020 (2:00pm – 4:00pm)

- Information Access & Hunter’s Hope Family Programs
- Leukodystrophy Care Network (LCN) Website & App
- Hunter’s Hope Family Programs
  - Wall of Fame
  - Equipment and Supply Exchange
  - Wish Gift
  - Other Family Support Programs
  - Kid Companion

Thursday Evening (6:00 pm)

- Balloon Release & Walk

Friday, July 24, 2020 (11:00am – 1:00pm)

- Family Sessions (Open to Affected Families Only)
  - Family Perspective

Lunch (1:00pm – 2:00pm)

Friday, July 24, 2020 (2:00pm – 4:00pm)

- Family Breakout Sessions
  - Moms – caring for a child
  - Moms – child in heaven
  - Dads – caring for a child
  - Dads – child in heaven
  - Siblings – Ages 13 and up

Friday Evening (7:00 pm)

- Talent Show
Tuesday, July 21, 2020 (10:00am – 3:00pm)

Lunch Break (12:00pm – 12:30pm)

Medical & Science Newborn Screening

These sessions will focus on Leukodystrophy Newborn Screening updates, advancements, guidelines and best practices, as well as the most current information regarding treatments and clinical trials.

State Labs Newborn Screening Updates (Group presentation by State)

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

Presenters
Presentations from state lab directors who are currently screening for Krabbe, ALD, and MLD and also those in the process of implementation.

Audience
These sessions will benefit NBS state lab staff, referral center providers, LCN Centers, and any provider or scientist focused on newborn screening, Leukodystrophies, and genetics, affected families, and industry partners.

Leukodystrophies NBS to Treatment

These sessions are organized by Leukodystrophy (LD) disease type: ALD, Krabbe, MLD, and other LDs. Each LD disease type has unique aspects that require specific guidelines. There are similarities across some of the LDs that may be discussed within one or more of the LD presentations.

Presenters
Up-to-date information will be shared by Leukodystrophy NBS Workgroup Members, who have been working diligently to address the gaps in the LD life-cycle process from NBS to Treatment.
Audience
Those who may want to attend these sessions include Leukodystrophy Care Network members, NBS state lab staff, referral center providers, LD medical providers and scientists focused on NBS, Leukodystrophies, and genetics, industry stakeholders, government stakeholders, medical students, affected families, and financial supporters.

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

Krabbe NBS Guidelines
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

EIKD NBS Case Study
A closer look into EIKD NBS patients identified and treated from Illinois, Ohio, Kentucky, and Missouri.

Krabbe NBS Patient Perspective Survey
One hundred seventy (170) families affected by Krabbe NBS and Krabbe Disease were surveyed about their experience and opinions regarding Krabbe NBS. What lessons does this provide for the medical and scientific community?

ALD NBS Guidelines
An update on best practices for clinicians and state labs involved in ALD NBS.

ALD NBS Parent Guidelines
An overview of the ALD NBS Parent Guidelines, created by the Aidan Jack Seeger Foundation.

MLD NBS Pilot and Next steps
An update on the NBS screening assay for MLD and outcomes of the MLD NBS pilots.
**CTX Guidelines**
An update on CTX guidelines for diagnosis and symptom management.

**Treatment for ALD, CTX, Krabbe and MLD**
Updates from the leading clinicians and scientists on current and emerging treatment options for patients affected by these diseases.

**Wednesday, July 22, 2020 (10:00am – 3:00pm)**

**Lunch Break (12:00pm – 12:30pm)**

**Leukodystrophy Care Network (LCN)**
For all Leukodystrophies the LCN embraces and promotes: the highest quality of life, an attitude that enhances and celebrates life, the affected individual’s abilities and potential, patient care as its primary focus, long-term and comprehensive care for the prevention of potential complications, a multidisciplinary team approach, exceptional standards of care, and a commitment to increasing value and continuously improving quality.

The key elements of the LCN’s strategy include: guided by family advocates to ensure patient centered care, learn from existing successful multidisciplinary care business models, integrated centers into established hospital systems, utilize existing resources where possible, world renowned experts in LDs and multidisciplinary care, standards of care, multidisciplinary team model, LD patient registry, ensure continuous excellence - LCN Advisory Committee, Involve government, industry, funders and other stakeholders throughout the process, promote education and growth.

**Presenters**
These presentations will be led by LCN Coordinating Center staff, LCN Steering Committee members, LCN Certified Center Directors, Care Coordinators, Clinical Practice Guideline Authors and workgroup members, Family Advocates, and other active members of the LCN.

**Audience**
Those who may want to attend these sessions include Leukodystrophy Care Network
members, LD medical providers, scientists, industry stakeholders, government stakeholders, medical students, affected families, and financial supporters.

**LCN Coordinating Center**
The LCN Coordinating Center is the administration and coordination arm of the LCN, working with all areas within the LCN to ensure affected individuals have access to the LCN and the most current information.

**LCN Certified Centers**
Center presentations to share with other LCN Centers the services and operational procedures provided. These presentations are not only intended to share best practices, but also inform Centers of how to best refer patients to other Centers based on specialty and/or location if needed.

**LCN Center Care Coordinators**
LCN Care Coordinators are Nurse Practitioners and RNs and are at the heart of all we do through the LCN. They will share updates from their workgroup and all they’ve accomplished over the past year.

**LCN Clinical Practice Guideline (CPG)**
Workgroups of medical providers and affected families have created a variety of CPGs over the past three years. Each group will provide a detailed presentation of their Guidelines in this educational session. Family advocates will share corresponding information about their Family Companion CPGs to inform providers of how to best educate their patients on these important Guidelines.

**LCN Leukodystrophy Data Registry (LDR)**

**LCN Telemedicine**
LCN Centers share how they have successfully implemented Telemedicine into their daily practice and how they have overcome obstacles such as third-party payer systems, technical issues, etc.

**LCN Resource Library**
The Resource Library is a website where both families and the medical and scientific
community can find helpful Leukodystrophy resources including: Clinical Practice Guidelines, Webinars, etc.

**LCN Family Perspective (Family Panel)**
Families share and inside look at what a family affected by Leukodystrophy faces each day, and how providers can best serve affected families.

**LCN Open Discussion – Priorities moving forward**

**Wednesday Evening (7:00 p.m.)**

**Welcome and Family Introductions**

The Kelly Family will welcome families to the Symposium.
A slideshow of each family registered will be shown along with each family introducing themselves.

**All are welcome to attend.**
Thursday, July 23, 2020 (11:00am – 1:00pm)

9:00 a.m.  **Prayer**
Prayer will be led by the Kelly Family and other families.

**Family Perspective - Medical**
The medical sessions will be a review of the prior day’s sessions, but from a family advocate perspective.

**Presenters**
These presentations will be led by LCN Medical Members, Family Advocates and Hunter’s Hope staff. This is an opportunity for all to understand these diseases from a family perspective.

**Audience**
Those who may want to attend these sessions include Leukodystrophy Care Network members, LD medical providers, scientists, industry stakeholders, government stakeholders, medical students, affected families, and financial supporters.

**State Labs NBS Updates Family Perspective (Family Panel)**
Review key updates from the Tuesday session from a family perspective.

**Leukodystrophies NBS to Treatment Family Perspective (Family Panel)**
Review key updates from the Tuesday session from a family perspective.

**LCN Family Perspective (Family Panel)**
Review key updates from the Wednesday session from a family perspective.

**Hunter’s Hope Krabbe NBS Update**
*Federally Recommended Uniform Screening Panel, Workgroups, and Council*
Hunter’s Hope staff and families will give an overview of the progress made and our upcoming plans for NBS.
State by State – Family Advocates
Learn from families who have advocated for Krabbe NBS in their states.

Lunch (1:00pm – 2:00pm)

Thursday, July 23, 2020 (2:00pm – 4:00pm)

Information Access & Hunter’s Hope Family Programs
These sessions will review and solicit input on Leukodystrophy information access and Hunter’s Hope Family Programs. All are encouraged to provide input with the aim of continuously improving information, access, and programs.

Presenters
These presentations will be led by Family Advocates and Hunter’s Hope staff.

Audience
Those who may want to attend these sessions include affected families, LCN Care Coordinators, industry stakeholders, government stakeholders, medical students, and financial supporters.

Leukodystrophy Care Network (LCN) Website & App
The Hunter’s Hope and LCN websites will be reviewed, along with the LCN app. Input from those attending will be discussed toward improving accessibility of family and medical information.

Hunter’s Hope Family Programs
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.

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.

**Kid Companion**
The Kid Companion is a wonderful “Clinical Practice Guideline” created by kids for kids. Ideas for more Kid Companion CPGs will be welcomed from the audience.

**Thursday Evening (6:00 pm)**

**Balloon Release & Walk**
Friday, July 24, 2020 (11:00am – 1:00pm)

Family Sessions (Open to Affected Families Only)

9:00 a.m.  
**Prayer**
Prayer will be led by the Kelly Family and other families.

**Family Perspective**

Affected families share what it’s really like to be a family affected by Leukodystrophy. How life as they knew it is changed. How extended family and friends react. Affected families from all stages of this unique journey will share and know they are not alone.

**Lunch (1:00pm – 2:00pm)**

Friday, July 24, 2020 (2:00pm – 4:00pm)

Family Breakout Sessions

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

- Moms – caring for a child
- Moms – child in heaven
- Dads – caring for a child
- Dads – child in heaven
- Siblings – Ages 13 and up

Friday Evening (7:00 pm)

**Talent Show**

Affected children and their siblings share their MANY talents – singing, dancing, jokes, and more! The Talent Show is open to all affected families and their friends. Join the fun!