

2018 Hunter's Hope Medical Symposium



HUNTER'S HOPE

## **2018 Medical Symposium**

### **Leukodystrophy Care Network**

Leukodystrophies |  
Newborn Screening | Research

July 16 - 19, 2018

Holiday Valley Resort – Ellicottville, NY

## 2018 Hunter's Hope Medical Symposium

### *Schedule Overview*

#### **Monday July 16, 2018**

Arrive	Holiday Valley Resort, Ellicottville NY
4:00pm	Registration Opens – Tamarack Club Lobby
7:15pm	Welcome Dinner – Sunrise, Holiday Valley Lodge

#### **Tuesday July 17, 2018**

7:00 – 8:00am	Breakfast – Sunrise, Holiday Valley Lodge
<b>Session I</b>	<b>Leukodystrophy Newborn Screening</b>
8:00am – 12:00pm	Fairway, 2 <sup>nd</sup> Floor, Holiday Valley Lodge
12:00 – 12:55pm	Lunch - Sunrise, Holiday Valley Lodge
<b>Session II</b>	<b>Standards of Care for Leukodystrophy NBS</b>
12:55 – 5:00pm	Fairway, 2 <sup>nd</sup> Floor, Holiday Valley Lodge
6:30pm	Dinner - Sunrise, Holiday Valley Lodge

#### **Wednesday July 18, 2018**

8:00 – 9:00am	Breakfast – Sunrise, Holiday Valley Lodge
<b>Session III</b>	<b>Leukodystrophy Care Network (LCN)</b>
9:00am – 4:15pm	Fairway, 2 <sup>nd</sup> Floor, Holiday Valley Lodge
12:15pm	Lunch - Sunrise, Holiday Valley Lodge
7:00pm	20 <sup>th</sup> Anniversary Celebration Dinner -Sunrise, Holiday Valley Lodge

\*LCN Care Coordinator Meeting – 4:30pm – 6:00pm, Birdie - Holiday Valley Lodge

#### **Thursday July 19, 2018**

8:00 – 9:30am	Breakfast - Sunrise, Holiday Valley Lodge
<b>Session IV</b>	<b>Presentations to Leukodystrophy Families</b>
9:30am – 2:30pm	Fairway, 2 <sup>nd</sup> Floor, Holiday Valley Lodge
11:45 – 12:45pm	Lunch - Sunrise, Holiday Valley Lodge

**Save the Date!**  
2019 Hunter's Hope  
Medical Symposium  
July 22-25, 2019

## 2018 Hunter's Hope Medical Symposium

Tuesday, July 17<sup>th</sup>

<b>7:00am</b>	<b>Breakfast</b>	<b>Sunrise, HV Lodge</b>
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### Session I and II – Fairway, 2<sup>nd</sup> Floor Holiday Valley Lodge

<b>8am – 12:00pm</b>	<b>Session I: Leukodystrophy Newborn Screening and Treatment</b>	<b>Fairway</b>
8:00 – 10:00am	<i>Leukodystrophy Newborn Screening (NBS) – State Updates</i>	
8:00 – 8:20am	New York	Joseph Orsini, PhD
8:20 – 8:35am	Missouri	Esperenza Font-Montgomery, MD
8:35 – 8:55am	Kentucky	Dietrich Matern, MD, PhD and Darrin Sevier
8:55 – 9:10am	Ohio	Margie Ream, MD, PhD
9:10 – 9:30am	Tennessee	George Dizikes, PhD
9:30 – 9:45am	Illinois	Khaja Basheeruddin, PhD
9:45 – 10:00am	MLD NBS	Michael Gelb, PhD
10:00 – 10:25am	Krabbe NBS Clinical Practice Guidelines (CPGs)	Jennifer Kwon, MD
10:25 – 12:00pm	<i>Leukodystrophy Treatment</i>	
10:25 – 10:55am	Krabbe Disease, MLD and ALD	Joanne Kurtzberg, MD
10:55 – 11:15am	Krabbe Disease	David Wenger, PhD
11:15 – 11:40am	ALD Clinical Trial	Florian Eichler, MD
11:40 – 12:00pm	Family Perspective	
<b>12:00 – 12:55pm</b>	<b>Lunch</b>	<b>Sunrise, HV Lodge</b>
12:55 – 5:00pm	<b>Session II: Standards of Care for Leukodystrophy NBS</b>	<b>Fairway</b>
12:55 – 1:15pm	Krabbe NBS CPGs for Late Onset Krabbe Disease (LOKD)	Joseph Orsini, PhD
1:15 – 1:30pm	Psychosine as 2 <sup>nd</sup> Tier Test for Krabbe NBS	Dietrich Matern, MD, PhD
1:30 – 1:45pm	High sensitivity GALC Assay	Michael Gelb, PhD
1:45 – 2:05pm	LOKD Cases Treated with Hematopoietic Stem Cell Transplantation	Joanne Kurtzberg, MD
2:05 – 2:25pm	Presentation of LOKD NBS Cases	Jennifer Kwon, MD
2:25 – 3:25pm	Recommended Follow Up for Possible LOKD Cases	
3:25 – 5:00pm	ALD NBS Follow Up	Ali Fatemi, MD, Elisa Seeger

<b>6:30pm</b>	<b>Dinner</b>	<b>Sunrise, HV Lodge</b>
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**Wednesday, July 18<sup>th</sup>**

<b>8:00 – 9:00am</b>	<b>Breakfast</b>	<b>Sunrise, HV Lodge</b>
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**Session III – Fairway, 2<sup>nd</sup> Floor Holiday Valley Lodge**

<b>9:00am – 4:15pm</b>	<b>Session III: Leukodystrophy Care Network (LCN)</b>	<b>Fairway</b>
9:00 – 9:15am	Welcome and Introduction	Anna Grantham
9:15 – 10:15am	Discussion of LCN Center Protocol for Patient Referrals	Catherine Becker, NP
	<b>Leukodystrophy Clinical Practice Guidelines (CPGs) – Overview and Updates</b>	
10:15 – 10:45am	Musculoskeletal	Sally Evans, MD
10:45 – 11:15am	Treatment	Joanne Kurtzberg, MD
11:15 – 11:45am	Neurology	Florian Eichler, MD
11:45 – 12:00pm	Respiratory	Geovanny Perez, MD
12:00 – 12:15pm	Diagnosis	Maura Ruzhnikov, MD
	Group Photo	

<b>12:15pm – 1:15pm</b>	<b>LUNCH</b>	<b>Fairway</b>
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1:15 – 2:45pm	CPG Breakout Sessions <ul style="list-style-type: none"> <li>•Musculoskeletal – Fairway</li> <li>•Treatment – Bunker, 2<sup>nd</sup> Floor</li> <li>•Neurology – Moonshadow, 1<sup>st</sup> Floor</li> <li>•Respiratory – Morning Star, 1<sup>st</sup> Floor</li> <li>•Diagnosis – Birdie, 2<sup>nd</sup> Floor</li> </ul>	
2:45 – 3:45pm	CPG Breakout Group Updates	CPG Lead Authors
3:45 – 4:15pm	Group Discussion	Florian Eichler, MD

4:30 – 6:00pm	LCN Care Coordinator Workgroup Meeting	Birdie – HV Lodge
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<b>7pm</b>	<b>Dinner – Please join us as we celebrate 20 years of Hunter's Hope and meet our Leukodystrophy families.</b>	<b>Sunrise, HV Lodge</b>
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**Thursday, July 19<sup>th</sup>**

<b>8:00am</b>	<b>Breakfast</b>	<b>Sunrise, HV Lodge</b>
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**Session IV – Fairway, 2<sup>nd</sup> Floor Holiday Valley Lodge**

<b>9:30 – 2:30pm</b>	<b>Leukodystrophy Care Network (LCN) Presentations to Leukodystrophy Families</b>	<b>Fairway</b>
9:30 – 9:40am	Welcome & LCN Overview	Jacque Waggoner and Anna Grantham
9:40 – 9:55am	MLD NBS	Michael Gelb, PhD
9:55 – 10:10am	Krabbe and ALD Newborn Screening (NBS) Overview	Joseph Orsini, PhD
10:10 – 10:25am	Krabbe NBS Clinical Practice Guidelines	Jennifer Kwon, MD
10:25 – 10:40am	Guidelines for ALD NBS	Ali Fatemi, MD
10:40 – 10:55am	LCN Centers	Stephanie Keller, MD
10:55 – 11:10pm	LCN Care Coordinators	Tara West, NP
11:10 – 11:45pm	Treatment	Joanne Kurtzberg, MD

<b>11:45 – 12:45pm</b>	<b>LUNCH</b>	<b>Fairway</b>
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12:45 – 1:45pm	Clinical Practice Guidelines <ul style="list-style-type: none"> <li>• Musculoskeletal</li> <li>• Treatment</li> <li>• Neurology</li> <li>• Respiratory</li> <li>• Diagnosis</li> </ul>	CPG Lead Authors
1:45 – 2:15	Advancements in Leukodystrophies	Amy Waldman, MD
2:15 – 2:30pm	ALD Clinical Trial	Florian Eichler, MD

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