



HUNTER'S HOPE

## **2016 Medical Symposium**

### **Leukodystrophies**

Newborn Screening | Research |  
Leukodystrophy Care Network  
July 25 - 27, 2016

2016 Family Symposium  
LCN Centers Presentations  
July 28, 2016

Holiday Valley Resort – Ellicottville, NY

## 2016 Hunter's Hope Medical Symposium Agenda

### *Schedule Overview*

#### **Monday July 25, 2016**

Arrive	Holiday Valley Resort, Ellicottville NY
7:30pm	Welcome Dinner – Sunrise, Holiday Valley Lodge

#### **Tuesday July 26, 2016**

7am – 8am	Breakfast – Sunrise, Holiday Valley Lodge
<b>Session I</b>	<b>Newborn Screening for Krabbe &amp; Similar Disorders</b>
8am – 12:30pm	Fairway, 2 <sup>nd</sup> Floor, Holiday Valley Lodge
12:30pm	Lunch - Sunrise, Holiday Valley Lodge
<b>Session II</b>	<b>Translational Science &amp; Leukodystrophies</b>
1:30pm – 5pm	Fairway, 2 <sup>nd</sup> Floor, Holiday Valley Lodge
6pm	Dinner - Sunrise, Holiday Valley Lodge

#### **Wednesday July 27, 2016**

7am – 8am	Breakfast – Sunrise, Holiday Valley Lodge
<b>Session III</b>	<b>Leukodystrophy Care Network (LCN) Clinical Guidelines Planning and Development</b>
8am – 12pm	Fairway, 2 <sup>nd</sup> Floor, Holiday Valley Lodge
12pm	Lunch - Sunrise, Holiday Valley Lodge
	<b>Leukodystrophy Care Network (LCN) Clinical Guidelines Planning and Development (Continued)</b>
1pm – 3:30pm	Fairway, 2 <sup>nd</sup> Floor, Holiday Valley Lodge
7pm	Dinner - Sunrise, Holiday Valley Lodge

# Save the Date!

2017 Hunter's Hope Medical Symposium  
July 17-19, 2017

## 2016 Hunter's Hope Medical Symposium Agenda

**Tuesday, July 26<sup>th</sup>**

<b>7am</b>	<b>Breakfast</b>	<b>Sunrise, HV Lodge</b>
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### Session I – Fairway, 2<sup>nd</sup> Floor HV Lodge

<b>8am – 12:30pm</b>	<b>Session I: Newborn Screening for Krabbe &amp; Similar Disorders – Moderator: Joseph Orsini</b>	<b>Fairway</b>
8:00 – 8:05am	Welcome	Larry Wrabetz
8:05 – 9:45am	<i>Update From States</i>	
8:05 – 8:25am	New York	Joseph Orsini
8:25 – 8:45am	Missouri	Patrick Hopkins
8:45 – 9:15am	Kentucky	Darrin Sevier Dietrich Matern
9:15 – 9:25am	Illinois	Barbara Burton
9:25 – 9:35am	Tennessee	George Dizikes
9:35 – 9:45am	Pennsylvania	Kelly Holland
9:45 – 12:15pm	<i>Improving Outcomes for Krabbe NBS</i>	
9:45 – 10:10am	Improving Outcomes for Krabbe NBS: An Evidence Based Approach	Jennifer Kwon
10:10 – 10:25am	A Family Perspective	
<b>10:25 – 10:35am</b>	<b>Break</b>	
10:35 – 10:55am	Long Term Follow Up for Survivors of Cord Blood Transplant for Krabbe Disease	Joanne Kurtzberg
10:55 – 11:15am	Bivariate Tool for Reducing False Positive NBS for Infantile Krabbe Disease	Randy Carter
11:15 – 11:40am	GALC Assay with Expanded Range; MLD Pilot	Michael Gelb
11:40am – 12:00pm	DUOC and Novel Cell Therapy Approach to Augment Cord Blood Transplant for Krabbe Disease	Joanne Kurtzberg
12:00 – 12:30pm	Discussion	

<b>12:30pm</b>	<b>Lunch</b>	<b>Sunrise, HV Lodge</b>
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<b>1:30 – 5:00pm</b>	<b>Session II: Translational Science &amp; Leukodystrophies – Moderator: Larry Wrabetz</b>	<b>Fairway</b>
1:30 – 1:40pm	Advances in Treatment of Lysosomal Diseases	Chet Whitley

## 2016 Hunter's Hope Medical Symposium Agenda

1:40 – 2:05pm	Ex vivo Gene Therapy for MLD	Alessandra Biffi
2:05 – 2:30pm	Mechanotransduction and Myelination	Laura Feltri
2:30 – 2:55pm	Cell Biology of GalC and Diagnosis	Daesung Shin
2:55 – 3:20pm	Cell Based Assay of Psychosine Reduction	Gustavo Maegawa
<b>3:20 – 3:35pm</b>	<b>Break</b>	
3:35 – 4:00pm	Ricin-fusion Proteins for Delivery to the Nervous System	Carole Cramer
4:00 – 4:25pm	Update on Combinatorial Treatment of Twitcher Mice	Mark Sands
4:25 – 4:50pm	BMT Plus ivAAVrh10 to Treat Twitcher Mice	David Wenger
4:50 – 5:00pm	Discussion	
<b>6pm</b>	<b>Dinner</b>	<b>Sunrise, HV Lodge</b>

## 2016 Hunter's Hope Medical Symposium Agenda

**Wednesday, July 27<sup>th</sup>**

<b>7am</b>	<b>Breakfast</b>	<b>Sunrise, HV Lodge</b>
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**Session III – Fairway, 2<sup>nd</sup> Floor HV Lodge**

<b>8am – 4pm</b>	<b>Session III: Leukodystrophy Care Network (LCN) Clinical Guidelines Planning and Development – Moderator: Jeff Leonard</b>	<b>Fairway</b>
8:00 – 8:15am	Welcome & Introduction	Jacque Waggoner
8:15 – 8:45am	Family Perspective	Jacque Waggoner Jeff Leonard
8:45 – 9:00am	LCN Clinical Issues Survey	Anna Grantham
9:00 – 10:00am	Prioritization of Clinical Issues for Respiratory, Musculoskeletal and Neurology & Next Steps	Jeff Leonard
10:00 – 11:30am	Development and Planning of Clinical Care Guidelines for Leukodystrophies	
11:30am – 12pm	Summary of Progress	Jeff Leonard

<b>12pm – 1pm</b>	<b>LUNCH</b>	
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1:00 – 2:45pm	Development and Planning of Clinical Care Guidelines for Leukodystrophies (Continued)	
2:45 – 3:15pm	Summary of Progress: Respiratory, Musculoskeletal and Neurology	
3:15 – 3:30pm	Next Steps	Jeff Leonard Jacque Waggoner

<b>7pm</b>	<b>Dinner</b>	<b>Sunrise, HV Lodge</b>
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## 2016 Hunter's Hope Medical Symposium Agenda

**Thursday, July 28<sup>th</sup>**

<b>8:00am</b>	<b>Breakfast – LCN Candidate Center Representatives</b>	<b>Bunker, HV Lodge</b>
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**Family Symposium, LCN Presentations and Panel– Fairway, 2<sup>nd</sup> Floor HV Lodge**

<b>10am</b>	<b>Leukodystrophy Care Network (LCN) Candidate Centers</b>	<b>Fairway</b>
10:00 – 10:10am	Welcome & Introduction	Jacque Waggoner
10:10 – 10:25am	Children's Hospital of Philadelphia	Amy Waldman
10:25 – 10:40am	Ann & Robert H. Lurie Children's Hospital of Chicago	Barbara Burton
10:40 – 10:55am	Kennedy Krieger Institute	Christopher Joseph
10:55 – 11:10am	Monroe Carell Jr. Children's Hospital at Vanderbilt	Kevin Ess
11:10 – 11:25am	Lucille Packard Children's Hospital/Stanford Hospital	Keith Van Haren
11:25 – 11:40am	University of Utah/Primary Children's Hospital	Gary Nelson
11:40 – 11:55am	Massachusetts General Hospital	Catherine Becker
11:55am – 12:25pm	Duke University Medical Center	Joanne Kurtzberg

<b>12:25 – 1:15pm</b>	<b>LUNCH</b>	
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1:15– 2:15pm	<b><i>LCN Candidate Center Panel Discussion</i></b> with - Waldman, Burton, Joseph, Ess, Van Haran, Nelson, Kurtzberg, Kwon and Kakish	
<b>2:15 – 2:30pm</b>	<b>BREAK</b>	
2:30 – 3:00pm	Improving Outcomes for Krabbe NBS	Jennifer Kwon
3:00 – 4:00pm	Leukodystrophy Research Advancements and Updates	Larry Wrabetz