



HUNTER'S HOPE

2017 Medical Symposium

Leukodystrophies

Newborn Screening | Research |

Leukodystrophy Care Network

July 17 - 20, 2017

Holiday Valley Resort – Ellicottville, NY

2017 Hunter's Hope Medical Symposium Agenda

Schedule Overview

Monday July 17, 2017

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

Tuesday July 18, 2017

8:00 – 9:00am	Breakfast – Sunrise, Holiday Valley Lodge
Session I	Early Diagnosis for Leukodystrophies through Newborn Screening
9:00am – 1:00pm	Fairway, 2 nd Floor, Holiday Valley Lodge
1:00 – 2:00pm	Lunch - Sunrise, Holiday Valley Lodge
Session II	Clinical Research and Treatments for Leukodystrophies
2:00 – 5:00pm	Fairway, 2 nd Floor, Holiday Valley Lodge
7:00pm	Dinner - Sunrise, Holiday Valley Lodge

Wednesday July 19, 2017

8:00 – 9:00am	Breakfast – Sunrise, Holiday Valley Lodge
Session III	Leukodystrophy Care Network (LCN) Clinical Practice Guidelines*
9:00am – 4:00pm	Fairway, 2 nd Floor, Holiday Valley Lodge
12:30pm	Lunch - Sunrise, Holiday Valley Lodge
7:00pm	Family Welcome Dinner - Sunrise, Holiday Valley Lodge

*Krabbe NBS Task Force Breakout – 8:30am – 12:30pm in Birdie (2nd floor, HV Lodge)

Thursday July 20, 2017

8:00 – 9:00am	Breakfast – Sunrise, Holiday Valley Lodge
	Family Symposium, LCN Presentations
10:00am – 4:00pm	Fairway, 2 nd Floor, Holiday Valley Lodge
12:45pm	Lunch - Sunrise, Holiday Valley Lodge

Save the Date!

2018 Hunter's Hope
Medical Symposium
July 16-18, 2018

2017 Hunter's Hope Medical Symposium Agenda

Tuesday, July 18th

8:00am	Breakfast	Sunrise, HV Lodge
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Session I – Fairway, 2nd Floor Holiday Valley Lodge

9am – 1:00pm	Session I: Early Diagnosis for Leukodystrophies through Newborn Screening	Fairway
9:00 – 9:10am	Welcome & Family Perspective	Jacque Waggoner
9:10 – 9:40am	New York	Joseph Orsini, PhD
9:40 – 10:10am	Missouri	Patrick Hopkins and Sharmini Rogers
10:10 – 10:25am	Tennessee	George Dizikes, PhD
10:25 – 10:40am	New Jersey	Shawn O'Leary
10:40 – 11:10am	Kentucky	Dietrich Matern, MD, PhD and Darrin Sevier
11:10 – 11:25am	Leukodystrophies: Newborn Screening and Post-Screening Analysis	Michael Gelb, PhD
11:25 – 11:35am	Bivariate analysis of GalC and Psychosine for Diagnosis of Infantile Krabbe Disease	Kabir Jalal, PhD
11:35 – 11:55am	Discussion on Role of Psychosine in Krabbe NBS	
11:55 – 12:25pm	Krabbe NBS Clinical Practice Guidelines	Jennifer Kwon, MD
12:25 – 1:00pm	Discussion, Next Steps	Joe Orsini, PhD
1:00 – 2:00pm	Lunch	Sunrise, HV Lodge
2:00 – 4:00pm	Session II: Clinical Research and Treatments for Leukodystrophies	Fairway
2:00 – 2:45pm	An Update on Cord Blood Therapies for Children with Genetic and Acquired Brain Disorders	Joanne Kurtzberg, MD
2:45 – 3:15pm	Lenti-D Gene Therapy for the Treatment of Cerebral Adrenoleukodystrophy: Interim Results from a Phase 2/3 Clinical Trial	Gary Fortin, PhD
3:15 – 4:00pm	Moving toward a Clinical Trial for Krabbe Gene Therapy	Maria Escolar, MD and David Wenger, PhD

7pm	Dinner	Sunrise, HV Lodge
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2017 Hunter's Hope Medical Symposium Agenda

Wednesday, July 19th

8:00am	Breakfast	Sunrise, HV Lodge
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Session III – Fairway, 2nd Floor Holiday Valley Lodge

9:00am – 4:00pm	Session III: Leukodystrophy Care Network (LCN) Clinical Practice Guidelines*	Fairway
9:00 – 9:15am	Welcome & LCN Update	Jacque Waggoner and Anna Grantham
9:15 – 10:00am	Family Perspective	LCN Steering Committee
	CPG Lead Authors Presentations	
10:00 – 10:15am	Treatment	Joanne Kurtzberg, MD
10:15 – 10:30am	Musculoskeletal	Sally Evans, MD
10:30 – 10:45am	Neurology	Florian Eichler, MD
10:45 – 11:00am	Respiratory	Richard Kravitz, MD
11:00 – 11:15am	Diagnosis	Adeline Vanderver, MD
11:15 – 11:30am	Plan for the Day	
11:30 – 12:30pm	Breakout Session I – Furthering CPG	
	Diagnosis CPG Workgroup	Bunker, 2nd Floor
	Musculoskeletal CPG Workgroup	Fairway
	Neurology CPG Workgroup	Morning Star, 1st Floor
	Respiratory CPG Workgroup	Fairway
	Treatment CPG Workgroup	Moonshadow, 1st Floor

*Krabbe NBS Task Force Breakout – 8:30am – 12:30pm in Birdie (2nd floor, HV Lodge)

12:30pm – 1:30pm	LUNCH	Fairway
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1:30 – 2:00pm	CPG Group Updates	
2:00 – 3:15pm	Breakout Session II – Furthering CPG, Next Steps and CPGs in LCN Centers	
3:15 – 4:00pm	CPG Group Updates and Discussion	

7pm	Dinner	Sunrise, HV Lodge
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2017 Hunter's Hope Medical Symposium Agenda

Thursday, July 20th

8:00am	Breakfast	Sunrise, HV Lodge
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Family Symposium, LCN Presentations – Fairway, 2nd Floor Holiday Valley Lodge

10:00 – 4:00pm	Leukodystrophy Care Network (LCN) Presentations to Leukodystrophy Families	Fairway
10:00 – 10:10am	Welcome & Introduction	Jacque Waggoner and Anna Grantham
10:10 – 10:25am	Duke Children's Hospital	Joanne Kurtzberg, MD
10:25 – 10:40am	Children's Hospital of Philadelphia	Amy Waldman, MD
10:40 – 10:55am	Massachusetts General Hospital	Florian Eichler, MD
10:55 – 11:10am	Monroe Carell, Jr. Children's Hospital at Vanderbilt	Kevin Ess, MD
11:10 – 11:25am	Ann & Robert H. Lurie Children's Hospital	Jennifer Rubin, MD
11:25 – 11:40am	Emory University/Children's Healthcare of Atlanta	Stephanie Keller, MD
11:40 – 11:55pm	Children's National Health System	Jamie Fraser, MD
11:55 – 12:45pm	Clinical Practice Guidelines	
	Treatment CPG	Joanne Kurtzberg, MD
	Musculoskeletal CPG	Sally Evans, MD
	Neurology CPG	Florian Eichler, MD
	Respiratory CPG	Richard Kravitz, MD

12:45 – 1:45pm	LUNCH	Fairway
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1:45 – 2:45pm	An Update on Cord Blood Therapies for Children with Genetic and Acquired Brain Disorders	Joanne Kurtzberg, MD
2:45 – 3:00pm	Break	
3:00 – 4:00pm	Leukodystrophy Research Advancements	Larry Wrabetz, MD