



HUNTER JAMES KELLY
RESEARCH INSTITUTE



2015 Medical Symposium

Leukodystrophies

Leukodystrophy Care Network | Clinical Research |
Newborn Screening | Basic Science Research

July 6 – 9, 2015

Holiday Valley Resort – Ellicottville, NY

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2015 Medical Symposium Agenda

Schedule Overview

Monday July 6, 2015

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

Tuesday July 7, 2015

7am – 8am	Breakfast – Sunrise Room, Holiday Valley Lodge
Session I	Leukodystrophy Care Network (Clinical Care)
8am – 12:15pm	Creekside Lodge
12:15pm	Lunch - Sunrise Room, Holiday Valley Lodge
Session II	Clinical Research Advancements
1:15pm – 5pm	Creekside Lodge
6pm	Dinner - Sunrise Room, Holiday Valley Lodge

Wednesday July 8, 2015

7am – 8am	Breakfast – Sunrise Room, Holiday Valley Lodge
Session III	Newborn Screening for Krabbe & Similar Disorders
8am – 10:30am	Creekside Lodge
Session IV	Long Term Follow Up for Krabbe NBS
10:30am – 12:30pm	Creekside Lodge
12:30pm	Lunch - Sunrise Room, Holiday Valley Lodge
Session IV	Long Term Follow Up for Krabbe NBS (Con't)
1:30pm – 4:15pm	Creekside Lodge
7pm	Dinner - Sunrise Room, Holiday Valley Lodge

Thursday July 9, 2015

7am – 8am	Breakfast – Sunrise Room, Holiday Valley Lodge
Session V	Translational Science
8am – 11am	Creekside Lodge
12pm	Lunch - Sunrise Room, Holiday Valley Lodge

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Tuesday, July 7th

7am	Breakfast	Sunrise, HV Lodge
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Session I – Creekside Lodge

8am – 12:15pm	Session I: Leukodystrophy Care Network (Clinical Care) – Moderator: Barbara Burton	Creekside Lodge
8:00 – 8:05am	Welcome	Larry Wrabetz Thomas Langan
8:05 – 8:35am	Leukodystrophy Care Network Overview (LCN)	Jacque Waggoner
8:35 – 9:05am	Newborn Screening for Neuromuscular Disorders: Ready or Not?	Jennifer Kwon
9:05am – 12:15pm	<i>Current Leukodystrophy Care Programs & Potential as LCN Centers</i>	
9:05 – 9:20am	Women and Children’s Hospital of Buffalo	Khalid Kakish Thomas Langan
9:20 – 9:35am	Children’s Hospital of Philadelphia	Amy Waldman
9:35 – 9:50am	Children’s National	Adeline Vanderver
9:50 – 10:05am	Children’s Hospital of Pittsburgh	Maria Escolar
10:05 – 10:20am	Duke University Medical Center	Joanne Kurtzberg
10:20 – 10:35am	Break	
10:35 – 10:50am	University of Minnesota Masonic Children’s Hospital	Paul Orchard, Wes Miller
10:50 – 11:05am	Kennedy Krieger Institute	Julie Cohen
11:05 – 11:20am	Lurie Children's Hospital	Barbara Burton
11:20 – 11:35am	Massachusetts General Hospital	Patricia Musolino Inna Tsvang
11:35 – 12:15pm	Discussion	

12:15pm	Lunch	Sunrise, HV Lodge
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1:15 – 5:00pm	Session II: Clinical Research -- Moderator: Chet Whitley	Creekside Lodge
1:15 – 1:40pm	GLIA	Adeline Vanderver
1:40 – 2:00pm	ALD Connect	Inna Tsvang
2:00 – 2:25pm	Current Studies on Leukodystrophies at the Hugo W. Moser Research Institute	Ann Moser
2:25 – 2:50pm	ALD Clinical Trial	Patricia Musolino
2:50 – 3:00pm	Break	
3:00 – 3:40pm	In-Utero Transplant/Clinical Trial/Long-term Outcomes for Transplant Patients	Joann Kurtzberg

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3:40 – 4:05pm	Long-term outcomes for transplant patients	Maria Escolar
4:05 – 4:25pm	Does Transplantation Modify Outcome in Symptomatic Krabbe Disease Patients?	Thomas Langan Randy Carter
4:25 – 4:45pm	Assessment of Psychosine and Galactocerebrosidase as Predictors of EIKD at the Time of Newborn Screening	Randy Carter Thomas Langan
4:45 – 5:00pm	Diffusion Tensor Imaging Analysis of a Canine Krabbe Brain	James Provenzale
5:00 – 5:15pm	Discussion	

6pm	Dinner	Sunrise, HV Lodge
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Wednesday, July 8th

7am	Breakfast	Sunrise, HV Lodge
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Session III – Creekside Lodge

8am – 10:30am	Session III: Newborn Screening for Krabbe & Similar Disorders – Moderator: Rodney Howell	Creekside Lodge
8:00 – 9:00am	State Updates	State Panel
8:00 – 8:20am	Newborn screening for Krabbe and other lysosomal storage disorders in NY	Joseph Orsini
8:20 – 8:40am	Missouri Krabbe Update	Patrick Hopkins Sharmini Rogers
8:40 – 8:50am	Illinois	George Dizikes
8:50 – 9:00am	New Jersey	Shawn O’Leary
9:00 – 9:20am	Post-analytical Interpretation of Newborn Screening Results for Krabbe Disease	Piero Rinaldo
9:20 – 9:40am	Psychosine as Biomarker for Krabbe	Dietrich Matern
9:40 – 10:00am	ALD Newborn Screening – NYS Experience	Michele Caggana
10:00 – 10:20am	What the Gelb Lab is Trying to Do to Improve Second Tier Analysis of Hits That Arise from Newborn Screening of Krabbe Disease	Michael Gelb
10:20am – 4:00pm	Session IV: Long-Term Follow Up, Krabbe NBS – Facilitator: Sally Jo Zuspan	
10:20 – 10:30am	Krabbe Consortium – First 10 years & Moving Ahead	Jennifer Kwon
10:30am – 12:30pm	Krabbe NBS Workshop – Description of Workshop Approach, Identification of Issues	

12:30 – 1:30pm	Lunch	Sunrise, HV Lodge
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1:30 – 4:15pm	Session IV: Long-Term Follow Up, Krabbe NBS – Facilitator: Sally Jo Zuspan (Con't)	Creekside Lodge
1:30 – 3:00pm	Krabbe NBS – Establishing Goals and Objectives	
3:00 – 3:15pm	Break	
3:15 – 4:00pm	Discussion and Next Steps	

7pm	Dinner	Sunrise, HV Lodge
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Thursday, July 9th

7am	Breakfast	Sunrise, HV Lodge
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Session V – Translational Science

8am – 11:00am	Session V: Translational Science – Moderator: Larry Wrabetz	Creekside Lodge
8:00 – 8:30am	Intrathecal AAV- Mediated Gene Transfer as a Translatable Treatment for Krabbe Disease	Steven Gray
8:30 – 9:00am	Combination therapy for Globoid Cell Leukodystrophy	Mark Sands
9:00 – 9:40am	Recent Advances in the Gene Therapy for Metachromatic and Globoid Leukodystrophies	Allesandra Biffi
9:40 – 10:10am	Natural History Study and Preliminary Assessment of Therapies in the Canine Model of Globoid Cell Leukodystrophy	Allison Bradbury
10:10 – 10:40am	Does Remyelination Protect Axons?	Ian Duncan
10:40 – 11:00am	Break	

12:00 – 1:30pm	Lunch	Sunrise, HV Lodge
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