

2019 Hunter's Hope
Medical Symposium
July 22 - 25, 2019

All Day		Monday, July 22nd - Registration in Tamarack Lobby - Dinner on your own...	
Start time	Finish Time	Topic	Speaker
Tuesday, July 23rd			
7:30		Breakfast	Fairway, 2nd Floor HV Lodge
		Leukodystrophy NBS and Treatment Moderator: Joe Orsini	Fairway, 2nd Floor HV Lodge
		State Updates	
8:30	8:50	New York Leukodystrophy NBS	Joseph Orsini, PhD
8:50	9:10	Missouri Krabbe Screening Updates	Tracy Klug, BS
9:10	9:30	Update on NBS for KD and ALD in KY and more on the laboratory diagnosis of KD	Dietrich Matern, MD, PhD
9:30	9:50	Newborn Screen for Leukodystrophies - The Illinois Experience	Khaja Beersherdin, PhD
9:50	10:05	Tennessee Leukodystrophy NBS	Lakisha Prowell, MT
10:05	10:20	Leukodystrophy Screening in Pennsylvania	Angela Gumby
10:20	10:30	Ohio Leukodystrophy NBS	Margie Ream, MD
10:30	10:50	Biochemical newborn screening for MLD and beyond using the most comprehensive, cost-effective, rapid, and robust platform to date	Michael Gelb, PhD
10:50	11:00	Break	
11:00	11:15	Outcomes after umbilical cord blood transplantation for infants with Early Infantile Krabbe Disease identified through the Kentucky, Missouri, and Ohio Newborn Screening Programs	Kristen Page, MD
11:15	12:15	Clinical Practice Guidelines for infants with LOKD diagnosed through NBS	Joseph Orsini, PhD and Joanne Kurtzberg, MD
12:15	12:30	Family Perspective/Panel	Fosters, Wilsons
12:30	1:30	Lunch	Sunrise, 1st Floor HV Lodge
1:30	1:50	Conditions for the BMT and iv AAVrh10-treatment of twitcher mice	David Wenger, PhD
1:50	2:20	DUOC, a novel cellular therapy for demyelinating diseases	Joanne Kurtzberg, MD
2:20	2:40	Intrathecal AAV9 gene therapy in the canine model of Krabbe disease	Allison Bradbury, PhD
2:40	3:10	ALD Guidelines	Ali Fatemi, MD
3:10	3:30	Adrenal Insufficiency Monitoring & Treatment in Adrenoleukodystrophy	Molly Regelmann, MD
3:30	3:50	Updated Safety and Efficacy Outcomes from an Ongoing Phase 2/3 Trial of Lenti-D Autologous Hematopoietic Stem Cell Gene Therapy for the Treatment of Cerebral Adrenoleukodystrophy	Andrew Dietz, MD
3:50	4:10	Takeda Phase 2b Trial of Intrathecal Enzyme Replacement Therapy in Late Infantile MLD	Barbara Burton, MD
4:10	4:30	CTX	Vivian Tucker, MD
	6:30	Dinner	Sunrise, 1st Floor, HV Lodge
	6:30	Krabbe NBS Advisory Council - Dinner	Birdie, 2nd Floor HV Lodge
	6:30	Care Coordinators - Dinner	Bunker, 2nd Floor HV Lodge

2019 Hunter's Hope
 Medical Symposium
 July 22 - 25, 2019

Start time	Finish Time	Topic	Speaker
		Wednesday, July 24	
7:30		Breakfast	Fairway, 2nd Floor HV Lodge
8:00		Welcome	Fairway, 2nd Floor HV Lodge
8:15	11:30	LCN Data Registry	Mike Dean, MD
11:30	11:45	Group Photo	
11:45	12:30	Lunch	Sunrise, 1st Floor HV Lodge
12:30	3:00	CPG Breakouts	
3:00	3:30	CPG Updates	
3:30	3:40	Discussion re: Insurance	Elizabeth Bailey-Sands, PT
3:40	3:50	Telemedicine	
3:50	4:00	Future topics for LCN, Closing Remarks	
7:00		Family Welcome Dinner	Fairway, 2nd Floor HV Lodge

**2019 Hunter's Hope
Medical Symposium
July 22 - 25, 2019**

Start	Finish	Topic	Speaker(s)
		Thursday, 7/25	
8:00	9:30	Breakfast	Sunrise, 1st Floor HV Lodge
9:30	9:45	Welcome and LCN Overview	Hunter's Hope, Fairway, 2nd Floor HV Lodge Moderator: Bob Rauner
9:45	10:05	Biochemical newborn screening for MLD and beyond using the most comprehensive, cost-effective, rapid, and robust platform to date	Michael Gelb, PhD
10:05	10:20	Takeda Phase 2b Trial of Intrathecal Enzyme Replacement Therapy in Late Infantile MLD	Barbara Burton, MD
10:20	10:50	Leukodystrophy Research and ALD NBS	Ali Fatemi, MD
10:50	11:00	Break	
11:00	11:15	Outcomes after umbilical cord blood transplantation for infants with Early Infantile Krabbe Disease identified through the Kentucky, Missouri, and Ohio Newborn Screening Programs	Kristen Page, MD
11:15	11:45	Umbilical Cord Blood Transplantation and cell therapy for Krabbe Disease and related Leukodystrophies	Joanne Kurtzberg, MD
11:45	12:05	Intrathecal AAV9 gene therapy in the canine model of Krabbe disease	Allison Bradbury, PhD
12:05	12:20	Adaptive Equipment	Elizabeth Bailey-Sands, PT
12:20	1:30	Lunch	Sunrise, 1st Floor HV Lodge
1:30	1:45	ALD Gene Therapy	Catie Becker, PNP
1:45	2:15	CPG Lead Author Panel	Presentation - 5 mins each
2:15	2:45	Care Coordinators Panel	
2:45	3:00	Break	
3:00	3:30	Leukodystrophy Data Registry	Mike Dean, MD
3:30	4:15	Family Panel - Advocate Organizations and Misc. Activity	Mike and Jenna Wallace, Elisa Seeger, Kristin Malfara, Anne Rugari, Lesa Brackbill, Christina Levasheff, Lauren Hammond