



## 2020 Annual Meeting and Patient Learning Academy

November 13, 2020 – 10:00 AM EST to 4:45 PM EST

- 10:00 AM-10:25 AM: Welcome: Updates on ALD and AMN  
*Dr. Florian Eichler, Massachusetts General Hospital*
- 10:30 AM-10:55 AM: Non-BMT Resolution of Cerebral ALD  
*Dr. Troy Lund, University of Minnesota*
- 11:00 AM-11:25 AM: Results of a Pilot Study of Vitamin D in ALD Boys  
*Dr. Keith Van Haren, Stanford University*
- 11:30 AM-11:55 AM: New Insights into Cerebral ALD  
*Dr. Joshua Bonkowsky, University of Utah*
- 12:00 PM-12:25 PM: ALD Newborn Screening Pilot Project  
*Dr. Eric Mallack, Weill Cornell Medicine*
- 12:30 PM-12:55 PM: Metabolic Rerouting of Very-Long-Chain Fatty Acid Synthesis Impacts Adrenoleukodystrophy  
*Dr. Stephan Kemp, Amsterdam University Medical Centers*
- 1:00 PM-1:40 PM: Patient Panel  
*Moderator: Tim Maguire*  
*Patients: Ken Dieffenbach, Jillian Smith, Sharon Mau, Barbara Machado*
- 1:45 PM-2:40 PM: Industry Panel  
*Moderator: Ben Lenail*  
*Presenters: bluebird bio, Inc., ImStem Biotechnology, Autobahn Therapeutics, Minoryx Therapeutics, Poxel, SwanBio Therapeutics, Viking Therapeutics*
- 2:45 PM-3:10 PM: Young Investigator Panel  
*Moderator: Dr. Ali Fatemi*  
*Dr. Jonanlis Ramirez Alcantara, Massachusetts General Hospital*  
*Dr. Isha Srivastava, Lucile Packard Children's Hospital*  
*Dr. Ashish Gupta, University of Minnesota*
- 3:15 PM-3:30 PM: Multi-Omic Approach to Identify Phenotypic Modifiers Underlying Cerebral Demyelination in X-Linked Adrenoleukodystrophy  
*Phillip Richmond, University of British Columbia*
- 3:30 PM-3:45 PM: Therapeutic Efficacy Potential of PXL770 - A Novel Direct AMPK-Activator and PXL065 – Deuterium-Stabilized R-Pioglitazone - for X-Linked Adrenoleukodystrophy  
*Dr. Jaspreet Singh, Henry Ford Hospital*
- 3:45 PM-4:10 PM: Gene Correction in Hematopoietic Stem Cells Restores White Matter Microvascular Function in Cerebral Adrenoleukodystrophy  
*Dr. Patricia Musolino, Massachusetts General Hospital*
- 4:15 PM-4:30 PM: Newborn Screening Update  
*Elisa Seeger*
- 4:30 PM-4:45 PM: ALD Connect Awards  
*Presented by Kathleen O'Sullivan-Fortin and Greg Benton*



## 2020 Annual Meeting and Patient Learning Academy

November 14, 2020 – 10:00 AM EST to 6:00 PM EST

10:00-10:25 AM Webinar format, chat Q&A	Welcome and Introduction to ALD Connect <i>Kathleen O'Sullivan-Fortin</i>				
10:30-10:55 AM Webinar format, chat Q&A	ALD Family Weekend: Building Community <i>Nic Adler</i>				
11:00-11:40 AM Webinar format, chat Q&A	More Than a Diagnosis: Living with ALD and the Mental Health Impact <i>Johanna Bond, LMHC</i>				
11:45-12:25 PM Live/Interactive	Breakout Sessions <i>Community Call Facilitators</i>				
	Asymptomatic/ Newborn Screening	Cerebral ALD	Men	Women	
12:30-1:10 PM Webinar format, chat Q&A	Endocrine Considerations in Adrenoleukodystrophy <i>Dr. Molly Regelman</i>				
1:15-1:40 PM Webinar format, chat Q&A	Genetic Counseling <i>Moderator: Felicity Emerson Deirdre Sumski, CGC Janis Sherwood</i>	Functional Considerations in Symptomatic Myelopathy Associated with ALD <i>Dr. Brian Wishart</i>	Women with ALD: Current Research, Prevention of Symptoms, Therapies for Symptoms <i>Dr. Nutan Sharma Dr. John Winkelman</i>		
1:45-2:25 PM Webinar format, chat Q&A	Education Supports for ALD Boys: IEPs and 504 Plans <i>Moderator: Kathleen O'Sullivan- Fortin Nicole Metsisto Mazer, Esq. Bill and Kate Groel</i>	Post-Transplant <i>Moderator: Dr. Troy Lund Julie Purschke</i>	Planning for the Future: Financial Planning <i>Alexandria Nadworny, CFP, CTFA Jesse Torrey</i>		
2:30 PM-2:55 PM Webinar format, chat Q&A	Grassroots Fundraising <i>Moderator: Greg Benton Chris and Katie Marino, X out ALD</i>		Multidisciplinary Care and ALD <i>Catherine Becker, RN/NP</i>		
3:00-3:55 PM Live/Interactive	Breakout Sessions <i>Community Call Facilitators</i>				
	Asymptomatic/Newborn Screening	Cerebral ALD	Men	Women	Industry Advisory Council
4:00-4:55 PM Webinar format, chat Q&A	Ask the Experts <i>Dr. Joshua Bonkowsky, Dr. Florian Eichler, Dr. Troy Lund, Dr. Molly Regelman, Dr. Keith Van Haren</i>				
5:00-6:00 PM Live/Interactive	Social Hour ALD Connect: 2 Truths and a Lie <i>Hosted by Jon Kuderer</i>				