



2020 ANNUAL MEETING & PATIENT LEARNING ACADEMY

FRIDAY NOVEMBER 13 & SATURDAY NOVEMBER 14, 2020

FRIDAY NOVEMBER 13: ANNUAL MEETING

TIME	PROGRAM <i>(Click on any Session Name for the Link to join!)</i>
<i>(All Times Shown EST)</i>	
FRIDAY 10:00 AM - 10:25 AM	<u>WELCOME: UPDATES ON ALD & AMN</u> <i>Dr. Florian Eichler, Massachusetts General Hospital</i>
FRIDAY 10:30 AM - 10:55 AM	<u>NON-BMT RESOLUTION OF CEREBRAL ALD</u> <i>Dr. Troy Lund, University of Minnesota</i>
FRIDAY 11:00 AM - 11:25 AM	<u>RESULTS OF A PILOT STUDY OF VITAMIN D IN ALD BOYS</u> <i>Dr. Keith Van Haren, Stanford University</i>
FRIDAY 11:30 AM - 11:55 AM	<u>NEW INSIGHTS INTO CEREBRAL ALD</u> <i>Dr. Joshua Bonkowsky, University of Utah</i>
FRIDAY 12:00 PM - 12:25 PM	<u>ALD NEWBORN SCREENING PILOT PROJECT</u> <i>Dr. Eric Mallack, Weill Cornell Medicine</i>
FRIDAY 12:30 PM - 12:55 PM	<u>METABOLIC REROUTING OF VERY-LONG-CHAIN FATTY ACID SYNTHESIS IMPACTS ADRENOLEUKODYSTROPHY</u> <i>Dr. Stephan Kemp, Amsterdam University Medical Centers</i>
FRIDAY 1:00 PM - 1:40 PM	<u>PATIENT PANEL</u> <i>Moderator: Tim Maguire</i> <i>Patients:</i> <i>Ken Dieffenbach, Jillian Smith</i> <i>Sharon Mau, Barbara Machado</i>

	<u>INDUSTRY PANEL</u>
FRIDAY 1:45 PM - 2:40 PM	<i>Moderator: Ben Lenail Presenters: bluebird bio, Inc., ImStem Biotechnology Autobahn Therapeutics, Minoryx Therapeutics Poxel, SwanBio Therapeutics Viking Therapeutics</i>
	<u>YOUNG INVESTIGATOR PANEL</u>
FRIDAY 2:45 PM - 3:10 PM	<i>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</i>
	<u>MULTI-OMIC APPROACH TO IDENTIFY PHENOTYPIC MODIFIERS UNDERLYING CEREBRAL DEMYELINATION IN X-LINKED ADRENOLEUKODYSTROPHY</u>
FRIDAY 3:15 PM - 3:30 PM	<i>Phillip Richmond, University of British Columbia</i>
	<u>THERAPEUTIC EFFICACY POTENTIAL OF PXL770 - A NOVEL DIRECT AMPK- ACTIVATOR and PXL065 - DEUTERIUM-STABILIZED R-PIOGLITAZONE - FOR X-LINKED ADRENOLEUKODYSTROPHY</u>
FRIDAY 3:30 PM - 3:45 PM	<i>Dr. Jaspreet Singh, Henry Ford Hospital</i>
	<u>GENE CORRECTION IN HEMATOPOIETIC STEM CELLS RESTORES WHITE MATTER MICROVASCULAR FUNCTION IN CEREBRAL ADRENOLEUKODYSTROPHY</u>
FRIDAY 3:45 PM - 4:10 PM	<i>Dr. Patricia Musolino, Massachusetts General Hospital</i>
	<u>NEWBORN SCREENING UPDATE</u>
FRIDAY 4:15 PM - 4:30 PM	<i>Elisa Seeger</i>
	<u>ALD CONNECT AWARDS</u>
FRIDAY 4:30 PM - 4:45 PM	<i>Presented by Kathleen O'Sullivan-Fortin and Greg Benton</i>

SATURDAY, NOVEMBER 14: PATIENT LEARNING ACADEMY

TIME	PROGRAM <i>(Click on any Session Name for the Link to join!)</i>
<i>(All Times Shown EST)</i>	
SATURDAY 10:00 AM - 10:25 AM	WELCOME & INTRODUCTION TO ALD CONNECT <i>Kathleen O'Sullivan-Fortin</i>
SATURDAY 10:30 AM - 10:55 AM	ALD FAMILY WEEKEND: BUILDING COMMUNITY <i>Nic Adler</i>
SATURDAY 11:00 AM - 11:40 AM	MORE THAN A DIAGNOSIS: LIVING WITH ALD AND THE MENTAL HEALTH IMPACT <i>Johanna Bond, LMHC</i>
SATURDAY 11:45 AM - 12:25 PM	<p align="center">BREAKOUT SESSIONS</p> <p align="center"><i>Community Call Facilitators</i></p> <p align="center">Breakout Room 1: Asymptomatic / Newborn Screening</p> <p align="center">Breakout Room 2: Cerebral ALD</p> <p align="center">Breakout Room 3: Men</p> <p align="center">Breakout Room 4: Women</p>
SATURDAY 12:30 PM - 1:10 PM	ENDOCRINE CONSIDERATIONS IN ADRENOLEUKODYSTROPHY <i>Dr. Molly Regelman</i>
SATURDAY 1:15 PM - 1:40 PM (3 Options)	<p align="center">GENETIC COUNSELING</p> <p align="center"><i>Moderator: Felicity Emerson Deirdre Sumski, GC Janis Sherwood</i></p> <p align="center">FUNCTIONAL CONSIDERATIONS IN SYMPTOMATIC MYELOPATHY ASSOCIATED WITH ALD</p> <p align="center"><i>Dr. Brian Wishart</i></p> <p align="center">WOMEN WITH ALD: CURRENT RESEARCH, PREVENTION OF SYMPTOMS, THERAPIES FOR SYMPTOMS</p> <p align="center"><i>Dr. Nutan Sharma Dr. John Winkelman</i></p>

SATURDAY 1:45 PM - 2:25 PM (3 Options)	<u>EDUCATION SUPPORTS FOR ALD BOYS: IEPs AND 504 PLANS</u>
	<i>Moderator: Kathleen O'Sullivan-Fortin Nicole Metsisto Mazer, Esq. Bill and Kate Groel</i>
	<u>POST-TRANSPLANT</u>
	<i>Moderator: Dr. Troy Lund Julie Purschke, Jillian Smith</i>
SATURDAY 2:30 PM - 2:55 PM	<u>GRASSROOTS FUNDRAISING</u>
	<i>Moderator: Greg Benton Chris and Katie Marino, X out ALD</i>
SATURDAY 3:00 PM - 3:55 PM	BREAKOUT SESSIONS
	<i>Community Call Facilitators</i>
	<u>Breakout Room 1: Asymptomatic / Newborn Screening</u>
	<u>Breakout Room 2: Cerebral ALD</u>
	<u>Breakout Room 3: Men</u>
SATURDAY 4:00 PM - 4:55 PM	<u>ASK THE EXPERTS</u>
	<i>Dr. Joshua Bonkowsky, Dr. Florian Eichler Dr. Troy Lund, Dr. Molly Regelman Dr. Keith Van Haren</i>
SATURDAY 5:00 PM - 6:00 PM	<u>SOCIAL HOUR: ALD Connect: 2 Truths and a Lie</u>
	<i>Hosted by Jon Kuderer</i>