



2021 Virtual Family Conference Friday, June 25



	Session Block A	Session Block B	Session Block C
9:00 AM	PMD <i>Dr. Nicole Wolf</i> <i>Dr. Davida Tonduti</i>	Partner Organization: VWM Consortium 9 AM to 1 PM Please see the VWM Agenda!	Partner Organizations: Cure MLD & the Calliope Joy Foundation 9 AM to 1 PM Please see the MLD Agenda!
10:30 AM	Canavan <i>Dr. Dominic Gessler</i>		
12:00 PM	Global Leukodystrophy Initiative <i>Dr. Adeline Vanderver</i> <i>Dr. Florian Eichler</i> <i>Dr. Ali Fatemi</i>		
1:00 PM	Meal Break <i>Messages from our Industry Partners</i>	Meal Break <i>Messages from our Industry Partners</i>	Meal Break <i>Messages from our Industry Partners</i>
2:30 PM	AGS <i>Dr. Adeline Vanderver</i>	VWM: Guanabenz Trial <i>Dr. Marjo van der Knaap</i> <i>Donna Skwirut</i>	Leukodystrophy Newborn Screening Action Network <i>Lesa Brackbill</i>
4:00 PM	CTX <i>Dr. Andrea DeBarber</i>	AARS/AARS2 and tRNA Synthetases <i>Dr. Adeline Vanderver</i> <i>Dr. Amena Smith</i> <i>Dr. Marc Engelen</i>	“Harnessing Grief” Book Discussion <i>Maria Kefalas</i> <i>Blyth Lord</i>
5:30 PM	Support Group: Caregivers of Adults Moderated Discussion	Support Group: Newly Diagnosed Families Moderated Discussion	PFDD Meetings 101: Patient Focused Drug Development and the FDA

Thank you to our partner organizations offering VWM and MLD content this year!



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This schedule is subject to change. Times listed above are in USA Central.

Start times:

*USA Central: 9 AM • USA Eastern: 10 AM • USA Mountain: 8 AM • USA Pacific: 7 AM
London, UK: 3 PM • Amsterdam, Netherlands: 4 PM • Sydney, AU: 12 AM 6/26*



2021 Virtual Family Conference Saturday, June 26



	Session Block A	Session Block B	Session Block C
9:00 AM	AMN <i>Dr. Ali Fatemi</i> <i>Dr. Florian Eichler</i> <i>Dr. Gerald Raymond</i>	MLC <i>Dr. Rogier Min</i> <i>Dr. Marjo van der Knaap</i>	Partner Organizations: KrabbeConnect 9 AM to 1 PM Please see the Krabbe Agenda!
10:30 AM	AMN and ALD Clinical Trials <i>Dr. Wolfgang Koehler</i> <i>Dr. Keith Van Haren</i>	4H <i>Dr. Genevieve Bernard</i> <i>Dr. Nicole Wolf</i>	
12:00 PM	ALD <i>Dr. Marc Engelen</i> <i>Dr. Stephan Kemp</i> <i>Dr. Troy Lund</i>	Alexander Disease <i>Dr. Albee Messing</i>	
1:00 PM	Meal Break <i>Upcoming Programs by the ULF</i>	Meal Break <i>Upcoming Programs by the ULF</i>	Meal Break <i>Upcoming Programs by the ULF</i>
2:30 PM	ALSP/HDLS <i>Dr. Florian Eichler</i> <i>Dr. Jennifer Orthmann-Murphy</i> <i>Heidi Edwards</i>	Rare Disease Clinical Trials <i>Dr. Keith Van Haren</i> <i>Angela Pontius</i>	Wheelchair Physical Therapy <i>Dr. Kathy Zackowski</i> <i>Jennifer Keller</i>
4:00 PM	Mental Health for Caregivers and Families <i>Cristol O'Loughlin</i> <i>Dr. Albert Freedman</i>	Wheelchair Sports and Team IMPACT <i>Chris Kaag</i> <i>Kiernan Mcghee</i>	Industry Update Pharmaceutical Panel Meeting Sponsors
5:30 PM	Support Group: Caregivers of Children Moderated Discussion	Support Group: Affected Adults Moderated Discussion	Support Group: Siblings of Affected People Moderated Discussion

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2021 Virtual Family Conference VWM: Friday, June 25



	Topic	Speaker
9:00 AM	Mission of the Consortium of Clinical VWM Experts	<i>Dr. Marjo van der Knaap</i>
9:10 AM	The Clinical Presentations of VWM	<i>Dr. Joshua Bonkowsky</i>
9:30 AM	Therapeutic Options in VWM	<i>Dr. Marjo van der Knaap</i>
9:50 AM	Approaches to Trials for Rare Diseases	<i>Dr. Peter van de Ven</i> <i>Dr. Pieter van Bokhoven</i>
10:20 AM	The Interface between Medicine and Industry	<i>Dr. Prisca Leferink</i>
10:35 AM	The Interface between Medicine and Patients & Families	<i>Dr. Genevieve Bernard</i>
10:50 AM	The Perspectives of VWM Families	<i>Donna Skwirut</i>
11:05 AM	Break	
11:30 AM	Inventory of Burden of the Disease: AGS as an Example	<i>Dr. Adeline Vanderver</i>
11:50 AM	Inventory of Burden of the Disease: MLD as an Example	<i>Dr. Ingeborg Krageloh</i>
12:10 PM	Experience from Previous Clinical Trials on Rare Neurological Disorders	<i>Dr. Enrico Bertini</i>
12:30 PM	VWM Registry	<i>PhD Student</i>
12:50 PM	General Discussion	

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2021 Virtual Family Conference MLD: Friday, June 25

	Topic	Speaker
9:00 AM	The Joy of Poop	<i>Dr. Sonal Patel Dr. Annique Hogan</i>
10:00 AM	MLD Global Voices, Storytelling, and the Power of Advocacy	<i>Georgina Morton Les Martin April Robson Kendra Riley George Drinkwater Erin Main</i>
12:00 PM	MLD Updates: HOME Study and Clinical Trials	<i>Dr. Paul Orchard Pamela Gavin</i>
12:30 PM	MLD Families Support Session	<i>Breakout Session Facilitated by Dr. Albert Freedman</i>

***Thank you to our partner organizations offering MLD content this year!
Learn more about Cure MLD at CureMLD.com and
the Calliope Joy Foundation at TheCalliopeJoyFoundation.org***



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2021 Virtual Family Conference Krabbe: Saturday, June 26

	Topic	Speaker
9:00 AM	KrabbeConnect Welcome and Organization Commitments	<i>Stacy Pike-Langefeld, President</i>
9:20 AM	KrabbeCURES Update and Plans	<i>Stacy Pike-Langefeld, President</i>
9:45 AM	Caregivers Experience on Advocating for Krabbe Disease NBS in their State	<i>Lesa Brackbill Kevin Cushman Bruce Kirbo</i>
10:15 AM	Navigating the Burden of This Question: How Many Children Do You Have?	<i>Jennifer Siedman, Director of Family, Patient and Industry Development at Courageous Parents Network</i>
10:45 AM	Industry Panel: How Does Patient/Caregiver Advisory Boards Aid Drug Development	<i>Panelists TBA</i>
11:15 AM	Break	
11:45 AM	Industry Presentations	<i>TBA</i>
12:15 PM	KrabbeConnect: Patient Assistance Program Role Out	<i>Anne Rugari, Vice President</i>
12:30 PM	Brain and Tissue Banking: How Does It Work and Is It Right for Me?	<i>Anne Rugari, Vice President</i>
1:00 PM	Community Advocate Award	<i>Wendy Zielen, Secretary</i>
1:15 PM	Closing Comments	<i>Stacy Pike-Langefeld, President</i>

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Learn more about KrabbeConnect at KrabbeConnect.org***



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