



## MYELIN PROJEKT

Gemeinsam gegen Multiple Sklerose und  
Leukodystrophien

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## Fifth International Symposium on Myelin Disorders

ALD Summit - Approaching Therapeutic Goals

Leipzig, June 4<sup>th</sup> - 5<sup>th</sup>, 2021

Registration  
[ukl-live.de/myelin-symposium](http://ukl-live.de/myelin-symposium)

INVITATION

### ORGANIZER

Myelin Projekt Deutschland e.V.  
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*“Fairy tales are more than true: not because they tell us that dragons exist, but because they tell us that dragons can be beaten”*

### Dear Colleagues

Times are changing. Many of you will remember our successful last personal Myelin Meeting, 2018 here in Leipzig, where we discussed our recent scientific work on our unrelentingly search for new and effective treatment options for patients with Leukodystrophy. Facing a devastating pandemic, we now are forced to meet online, which surely is no real substitute for a personal discussion; however, we all feel an urgent need for one-on-one dialogues and exchange of ideas as a motivating source of our work.

This is especially true for X-linked Adrenoleukodystrophy (X-ALD) where significant treatment progress has been achieved, maybe not strong enough to beat the dragon yet, but substantially weaken its power. For this reason, we decided to focus our Symposium on X-ALD, addressing all aspects of this disease, which is by far the most frequent genetic white matter disease in children and young adults.

On behalf of our local organizing committee, our co-operating partners “Myelin Project, Germany”, “European Leukodystrophy Association (ELA), Germany” and with the generous support of partners from the pharmaceutical industries, I cordially like to invite you to join us online.

We wish to bring together physicians and scientists from basic science and clinical research addressing both, the more general aspects of myelin biology and myelin pathology as well as its relevance for our daily clinical practice. This time, we will focus on X-ALD since emerging progress in newborn-screening, cell-based therapies and medical treatments emerged that needs to be discussed further.

On behalf of the University of Leipzig Medical Center and our local Organizing Committee I am happy to invite you to this first Online – Symposium.

Let’s beat the dragon!



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### Friday, June 4<sup>th</sup> 2021

16:30 - 16:40	Opening Remarks, Welcome to the Meeting	Joseph Claßen Leipzig, GER
16:40 - 17:00	Introduction to the Meeting	Wolfgang Köhler Leipzig, GER
Session I	<b>Keynote Lecture</b> Chair: Wolfgang Köhler	
16:50 - 17:30	Therapy Developments in Leukodystrophies	Marjo van der Knaap Amsterdam, NL
Session II	<b>Pathogenetic Aspects in X-ALD</b> Chairs: Alfonso Barrantes-Freer, Florian Then Bergh	
17:30 - 17:45	Concurrent Axon and Myelin Destruction Differentiates X-ALD from Multiple Sclerosis	Caroline Bergner Leipzig, GER
17:45 - 18:00	Metabolic Re-routing of Very-Long-Chain Fatty Acid Synthesis Impacts Adrenoleukodystrophy	Stephan Kemp Amsterdam, NL
18:00 - 18:15	New Insights in Cerebral Adrenoleukodystrophy	Josh Bonkowsky Salt Lake City, USA
18:15 - 18:45	DISCUSSION	
18:45 - 19:00	COFFEE - BREAK	
Session III	<b>Therapeutic Options, Part 1</b> Chairs: Jörn Köhl, Florian Eichler	
19:00 - 19:15	The Potential of Histone Deacetylase Inhibitors to Halt the Inflammatory Demyelination in Patients with Cerebral ALD	Johannes Berger Vienna, A
19:15 - 19:30	Is there a Role for VLCFA Normalization?	Marco Cappa Roma, I
19:30 - 19:45	Lipid Metabolism Disturbances in X-ALD Beyond VLCFA	Aurora Pujol Barcelona, ESP
19:45 - 20:00	Leriglitazone in AMN - Results from a Phase 2/3 Clinical Study	Wolfgang Köhler Leipzig, GER
20:00 - 20:15	DISCUSSION	

### Saturday, June 5<sup>th</sup> 2021

Session IV	<b>Monitoring Disease Progression</b> Chairs: Marjo van der Knaap, Johannes Berger	
16:00 - 16:15	Disease Progression in Male and Female AMN - Lessons Learned from Natural History Studies	Irene Huffnagel Amsterdam, NL
16:15 - 16:30	Measuring Severity of Myelopathy in X-ALD	Marc Engelen Amsterdam, NL
16:30 - 16:45	Applying Research to Medical Needs: Neurofilament Light Chain (NfL) Protein as a Biomarker for X-linked ALD	Isabelle Weinhofer Vienna, A
16:45 - 17:00	Biomarkers in AMN -The World Beyond NfL	Ali Fatemi Baltimore, USA
17:00 - 17:30	DISCUSSION	
17:30 - 17:45	COFFEE - BREAK	
Session V	<b>Therapeutic Options, Part II</b> Chairs: Charles Peters, Basil Sharrack	
17:45 - 18:00	Allogeneic Hematopoietic Stem Cell Transplantation in Childhood Cerebral Adrenoleukodystrophy	Troy Lund Minneapolis, USA
18:00 - 18:15	Allogeneic Hematopoietic Stem Cell Transplantation in Adulthood Cerebral ALD - The Leipzig Leukodystrophy Center Experience	Georg Franke Leipzig, GER
18:15 - 18:30	Gene Therapy in Cerebral ALD	Jörn Köhl Leipzig, GER
18:30 - 18:45	Gene Therapy in AMN	Florian Eichler Boston, USA
18:45 - 19:15	DISCUSSION	
19:15 - 19:30	Summary of the Meeting and Take-Home- Messages	Wolfgang Köhler Leipzig, GER
19:30	END OF THE SCIENTIFIC SYMPOSIUM	