



ELA families-scientists meeting 2024

Scientific Preliminary Program

Need to be confirmed

Day 1 - Saturday April 6th

Introduction Guy Alba, president of ELA International

2 PM CET

Adrenoleukodystrophy ALD - Adrenomyeloneuropathy AMN

2:15 PM

Stephan KEMP (The Netherlands): Newborn screening for X-linked adrenoleukodystrophy

Florian EICHLER (USA): Benefits and risks of *ex vivo* gene therapy for childhood cerebral adrenoleukodystrophy

Elise YAZBECK (France): Interim results of the open-label study, NEXUS, about the efficacy of leriglitazone in the treatment of pediatric cerebral adrenoleukodystrophy

Lisa SCHAFER (Germany): Multi-professional online care for symptomatic women with ALD - Results of the SMART-ALD study

Fanny MOCHEL (France): X-adrenoleukodystrophy in French adult patients: new treatments, new monitoring

Questions & Answers

BREAK

4:25 PM

Genetic and undetermined leukodystrophies - Pelizaeus-Merzbacher Disease PMD and others hypomyelinating leukodystrophies

4:40 PM

Françoise FIGUET (France): Genetics basics, heredity and therapy of undetermined leukodystrophies

Nicole WOLF (The Netherlands): Pelizaeus-Merzbacher disease: news

Vivi HEINE (The Netherlands): 4H patient brain organoids to study disease mechanisms

Geneviève BERNARD (Canada): RNA polymerase III-related hypomyelinating leukodystrophy

Pedro BRITES (Portugal): Myelin defects caused by a deficiency in Slc44a1, the choline transporter like-1

Questions & Answers

END of day 1

6:50 PM



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Day 2 - Sunday April 7th

Introduction Guy Alba, *president of ELA International*

2 PM CET

Megalencephalic leukoencephalopathy with subcortical cysts MLC, CACH syndrome VWM, Alexander and Canavan diseases

2:15 PM

Raul ESTEVEZ (Spain): Molecular biology of MLC: looking for therapies

Martine COHEN-SALMON (France): Involvement of the cerebrovascular system in MLC

Marjo van der KNAAP (The Netherlands): Update on vanishing white matter / CACH

Elly HOL (The Netherlands): Alexander Disease: why research is crucial to understand the disease and to work towards a cure

Annette BLEY (Germany): Canavan Disease: Basics and updates

Questions & Answers

BREAK

4:25 PM

Aicardi-Goutières Syndrome AGS, Metachromatic leukodystrophy MLD, Krabbe disease and Zellweger spectrum disorders

4:40 PM

Adeline VANDERVER (USA): Update on Aicardi-Goutières Syndrome (*to be confirmed*)

Soumeya BEKRI (France): Newborn screening for MLD: a global perspective

Caroline SEVIN (France): Therapeutic options for Metachromatic Leukodystrophy and Krabbe disease: where are we?

Femke KLOUWER (The Netherlands): Zellweger Spectrum Disorders

Questions & Answers

END of day 2

6:50 PM