ELA Families/Scientists meeting
ELA 2023
Tentative Scientific Program

Day 1 - Saturday April 15th

Introduction by Guy Alba, *President of ELA International* 2 PM CET

Adrenoleukodystrophy (ALD) - Adrenomyeloneuropathy (AMN) 2:15 PM

Isabelle WEINHOFER (Austria): A blood test to track brain damage: Biomarker-based risk prediction for the onset of cerebral ALD

Lisa SCHAFER (Germany): Clinical symptoms and quality of life in women with adrenoleukodystrophy

Florian EICHLER (USA): *to be determined*

Wolfgang KOEHLER (Germany): Lessons learned from a first international controlled clinical trial with Leriglitazone in men with Adrenomyeloneuropathy

Elise YAZBECK (France): *to be determined*

Questions & Answers

BREAK 4:25 PM

Genetic and undetermined leukodystrophies - Pelizaeus-Merzbacher Disease (PMD) - POLR3-HLD 4H 4:40 PM

To be determined: genetic for unknown leukodystrophies

Nicole WOLF (The Netherlands): Hypomyelination – what's new?

Vivi HEINE (The Netherlands): Cortical interneuron involvement in 4H leukodystrophy

Noémie HAMILTON (UK): Modelling RNASET2 leukodystrophy in zebrafish to develop transformative therapies

Questions & Answers **6:20 PM**

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Day 2 - Sunday April 16th

Introduction by Guy Alba, *President of ELA International* 2 PM CET

Megalencephalic leukoencephalopathy with subcortical cysts (MLC), CACH syndrome (VWM), Canavan and Alexander diseases 2:15 PM

Elena AMBROSINI (Italy): Molecular defects in MLC disease: how basic research can help finding therapeutic strategies

Marjo VAN DER KNAAP (The Netherlands): Update on vanishing white matter VWM - CACH syndrome

Matthias ECKHARDT (Germany): Does the neuropeptide NAAG plays a role in the pathogenesis of Canavan disease?

Angela GRITTI and Vasco MENEGHINI (Italy): Development of editing technologies to treat Alexander disease

Questions & Answers **3:55 PM**

BREAK 4:25 PM

Aicardi-Goutières Syndrome (AGS), Zellweger spectrum disorders, Krabbe disease and Metachromatic leukodystrophy (MLD) 4:40 PM

Marie-Louise FRÉMOND (France): Follow-up of an ELA-funded trial in Aicardi-Goutières syndrome

Femke KLOUWER (The Netherlands): Zellweger spectrum disorders

Marco CECCHINI and Ambra DEL GROSSO (Italy): Nanomedicine and Autophagy modulation in the mouse model of Krabbe disease

Caroline SEVIN (France): *to be determined*

Questions & Answers **6:20 PM**

END **6:50 PM**