



## ELA families-scientists meeting 2022 Scientific Program

### Day 1 - Saturday March 26th

**Introduction** Guy Alba, *president of ELA International* 2 PM

#### **Adrenoleukodystrophy ALD - Adrenomyeloneuropathy AMN**

*To be confirmed:* Newborn screening for ALD 2:15 PM

Florian EICHLER (USA): Upcoming trials for women with ALD and men with AMN 2:35 PM

Wolfgang KOEHLER (Germany): Improving quality of life in women with X-ALD 2:55 PM

Caroline SEVIN (France): Pediatric cerebral ALD 3:15 PM

Jean-Hugues DALLE (France): Main results of cell and gene therapy in the treatment of X-linked cerebral adrenoleukodystrophy 3:35 PM

**Questions & Answers** 3:55 PM

**BREAK** 4:25 PM

#### **Genetic and undetermined leukodystrophies - Pelizaeus-Merzbacher Disease PMD - POLR3-HLD 4H**

*To be confirmed:* Genetic and undetermined leukodystrophies 4:40 PM

David ROWITCH (UK): Advanced diagnosis, functional genomics and new treatments for Pelizaeus-Merzbacher Disease (PMD) 5:00 PM

Nicole WOLF (The Netherlands): Pelizaeus-Merzbacher disease – new aspects 5:20 PM

Geneviève BERNARD (Canada): POLR3-HDL 4H leukodystrophies 5:40 PM

Vivi HEINE (The Netherlands): A patient stem cell-based research platform for leukodystrophies 6:00 PM

**Questions & Answers** 6:20 PM

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## ELA families-scientists meeting 2022 Scientific Program

### Day 2 - Sunday March 27th

**Introduction** Guy Alba, *president of ELA International* 2 PM

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

Assumpció BOSCH (Spain): Preclinical gene therapy for megalencephalic leukoencephalopathy with subcortical cysts by specific glial transduction 2:15 PM

Marjo van der KNAAP (The Netherlands): Update on vanishing white matter VWM - CACH syndrome 2:35 PM

*To be confirmed:* Canavan disease 2:55 PM

Elly HOLL (The Netherlands): EJP Alexander disease 3:15 PM

Angela GRITTI & Vasco MENEHINI (Italy): Exploring novel gene editing technologies as potential treatments for Alexander's disease 3:35 PM

**Questions & Answers** 3:55 PM

**BREAK** 4:25 PM

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

Yannick CROW (UK): Aicardi-Goutières Syndrome - an update 4:40 PM

Marc ENGELEN (The Netherlands): Zellweger spectrum disorders: standards of care 5:00 PM

Maria ESCOLAR (USA): Krabbe disease 5:20 PM

Caroline SEVIN (France): Metachromatic leukodystrophy (MLD) 5:40 PM

Francesca FUMAGALLI (Italy): Gene Therapy: updates and future perspectives on the first approved treatment for early onset metachromatic leukodystrophy 6:00 PM

**Questions & Answers** 6:20 PM

**END** 6:50 PM

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