



ELA families-scientists meeting 2021 Scientific Program

March 27-28, 2021
Online meeting

PATHOLOGIES	WORKSHOPS	TIME (Paris CET)
Adrenoleukodystrophy - ALD	1	Saturday March 27 th – 2PM
Adrenomyeloneuropathy - AMN	1	Saturday March 27 th – 2PM
Aicardi-Goutières syndrome - AGS	4	Sunday March 28 th - 4:45PM
Alexander disease	3	Sunday March 28 th - 2PM
CACH - VWM	3	Sunday March 28 th - 2PM
Canavan disease	3	Sunday March 28 th - 2PM
H-ABC syndrome	2	Saturday March 27 th – 4:45PM
Krabbe disease	3	Sunday March 28 th - 2PM
Metachromatic Leukodystrophy - MLD	3	Sunday March 28 th - 2PM
Megalencephalic leukoencephalopathy with subcortical cysts - MLC	3	Sunday March 28 th - 2PM
Neonatal adrenoleukodystrophy - NALD	4	Sunday March 28 th - 4:45PM
Pelizaeus-Merzbacher disease - PMD	2	Saturday March 27 th – 4:45PM
Pelizaeus-Merzbacher disease like - PMD-like (MTC8)	2	Saturday March 27 th – 4:45PM
Pol R III – 4H	2	Saturday March 27 th – 4:45PM
Refsum disease	4	Sunday March 28 th - 4:45PM
Spastic paraplegia type 2	2	Saturday March 27 th – 4:45PM
Undetermined leukodystrophies - ULD	4	Sunday March 28 th - 4:45PM
VWM - CACH	3	Sunday March 28 th - 2PM
Zellweger syndrome	4	Sunday March 28 th - 4:45PM

With the support of





ELA families-scientists meeting 2021 Scientific Program

(to be confirmed)

Workshop 1. ALD - AMN

Saturday March 27th - 2PM - 4:30PM

Florian Eichler (USA) Moderator: Introduction and updates for X-linked Adrenoleukodystrophy

Johannes Berger (Austria): Monitoring brain integrity using blood derived biomarkers

Josh Bonkowsky (USA): Screenable Zebrafish Models for Leukodystrophy Therapeutics

Stephen Kemp (The Netherlands): Metabolic rerouting of very-long-chain fatty acid synthesis impacts adrenoleukodystrophy

Caroline Sevin (France): MIN-102 trial in children with cerebral ALD

Wolfgang Koehler (Germany): Results from a multinational clinical treatment study in adrenomyeloneuropathy.

Workshop 2. PMD - PMD-like (MCT8) - POLR11-4H - H-ABC and other hypomyelinating leukodystrophies

Saturday March 27th - 4:45PM - 7:15PM

Geneviève Bernard (Canada) Moderator: RNA polymerase III-induced leukodystrophy: research update

Vivi Heine (The Netherlands): A 4H patient stem cell model to study neuronal abnormalities

Nicole Wolf (The Netherlands): Pelizaeus-Merzbacher disease – possible treatment avenues

David Rowitch (United-Kingdom): Updates on PMD care and research

Workshop 3. MLC - VWM/CACH Syndrome - Alexander disease- Canavan disease and other vacuolating leukodystrophies - MLD - Krabbe disease

Sunday March 28th - 2PM - 4:30PM

Raul Estevez (Spain) Moderator: Reversibility in Megalencephalic leukoencephalopathy: molecular mechanisms and therapeutics

Marjo van der Knaap (The Netherlands): Update on vanishing white matter research and trial

Elly Hol (The Netherlands): European Joint Program and Alexander disease research: Mini-brains in a dish to study brain diseases

Matthias Klugmann (Germany): on Canavan leukodystrophy

Francesca Fumagalli (Italy): First gene therapy for the treatment of MLD

Françoise Piguet (France): Intravenous gene therapy for metachromatic leukodystrophy: proof of concept in the mouse model of the pathology

With the support of





ELA families-scientists meeting 2021 Scientific Program

Marco Cecchini (Italy): Nanoparticle-driven enzyme replacement therapy for the mouse model of Krabbe Disease

Workshop 4. AGS - Zellweger spectrum disorders - Genetic and undetermined leukodystrophies Sunday March 28th - 4:45PM - 7:15PM

Adeline Vanderver (USA) Moderator: Updates on Aicardi-Goutières Syndrome care and treatment

Marc Engelen (The Netherlands): on Zellweger spectrum disorders

Nathalie Cartier (France) & Françoise Piguet (France): Genetic and undetermined leukodystrophies

With the support of

