

2025 ULF Scientific Symposium Agenda

June 26, 2025
Subject to Change

Time (Central)	Title	Presenter
All Day	Paul A. Watkins Excellence in Research Poster Presentation Viewing (Arrowhead)	
8:00 AM - 08:15 AM	Introduction	Geneviève Bernard, MD, MSc, FRCPc McGill University View Bio
8:15 AM - 08:45 AM	"A Novel Leukodystrophy"	Marjo van der Knaap, MD, PhD Amsterdam University Medical Center/ Emma Children's Hospital View Bio
8:45 AM - 09:15 AM	"Prognostic Factors in Alexander Disease Type I"	Davide Tonduti, MD, PhD Vittore Buzzi Children's Hospital, Milan View Bio
9:15 AM - 09:45 AM	"Outcomes of Allogeneic Hematopoietic Stem Cell Transplantation in Late-juvenile and Adult Metachromatic Leukodystrophy Compared with Untreated Patients – a Multicenter Registry-based Study"	Daphne Schoenmakers, MD, PhD Student MLD Amsterdam UMC
9:45 AM - 10:00 AM	Break	
10:00 AM - 10:30 AM	"Amyloid Self-Assemblies of Sphingolipids - Pathogenic Agents in Globoid Cell Leukodystrophy"	Daniel Segal, PhD George S. Wise Faculty of Life Sciences Tel-Aviv University View Bio
10:30 AM - 11:00 AM	"Unraveling EPRS1-Related Leukodystrophy: Genetic, Clinical, and Pathophysiological Insights"	Alexandra Chapleau, PhD Neuroscience Student McGill University Health Center
11:00 AM - 11:30 AM	"Understanding Polr3-related Disease: Lessons from Polr3a mutant mice"	Ian M. Willis, PhD Albert Einstein College of Medicine New York View Bio
11:30 AM - 12:00 PM	"Investigations into the Pathogenesis of PMLD"	Charles K. Abrams, MD, PhD UI Health, University of Illinois Chicago View Bio
12:00 PM - 01:00 PM	Lunch in Burnham's Buffet, located in the lobby of the hotel	
1:00 PM - 01:30 PM	"Clinical Genetic Testing Returned Negative – What Next?"	Tomi Pastinen, MD, PhD Children's Mercy View Bio
1:30 PM - 02:00 PM	"Death and Gene Therapy for Leukodystrophies"	Joshua Bonkowsky, MD, PhD Primary Children's Center for Personalized Medicine View Bio
2:00 PM - 02:30 PM	"Biomarkers in the Diagnosis and Management of Aicardi Goutieres Syndrome"	Adeline Vanderver, MD Children's Hospital of Philadelphia Perelman School of Medicine at the University of Pennsylvania View Bio
2:30 PM - 02:45 PM	Break	
2:45 PM - 03:15 PM	"Myelin Lipid Composition and Catabolism in Adrenoleukodystrophy"	Nadav Weinstock, MD, PhD John Hopkins University View Bio

3:15 PM - 03:45 PM	“Can We Improve Rehabilitation for Women with X-ALD?”	Jennifer Keller, MS, PT Kennedy Krieger Institute View Bio
3:45 PM - 04:15 PM	“CTX: From Small Molecule Drugs to Gene-Based Therapies.”	Yedda Li, MD, PhD Massachusetts General Hospital Brigham and Women’s Hospital View Bio
4:15 PM - 04:30 PM	Closing	Geneviève Bernard, MD, MSc, FRCPC McGill University View Bio
4:30 PM - 06:30 PM	Paul A. Watkin's Excellence in Research Poster Presentations and Reception	All are welcome
6:00 PM	Dinner located in the Red Oak Ballroom	