



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 Full Professor, Departments of Neurology and Neurosurgery, Pediatrics, and Human Genetics, McGill University Pediatric Neurologist, Department of Specialized Medicine, Division of Medical Genetics, McGill University Health Center Scientist, Child Health and Human Development, RI MUHC Chair, ULF Medical and Scientific Advisory Board
8:15 AM - 08:45 AM	“A Novel Leukodystrophy”	Marjo van der Knaap, MD, PhD Professor of Child Neurology Amsterdam University Medical Center Department of Pediatrics / Child Neurology Amsterdam Leukodystrophy Center Emma Children’s Hospital
8:45 AM - 09:15 AM	“Prognostic Factors in Alexander Disease Type I”	Davide Tonduti, MD, PhD Vittore Buzzi Children’s Hospital, Milan
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 Shmunis School of Biomedicine and Cancer Research, George S. Wise Faculty of Life Sciences Tel-Aviv University
10:30 AM - 11:00 AM	“Unraveling EPRS1-Related Leukodystrophy: Genetic, Clinical, and Pathophysiological Insights”	Alexandra Chapeau, 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
11:30 AM - 12:00 PM	“Connexin 47 Related Leukodystrophy”	Charles K. Abrams, MD, PhD UI Health, University of Illinois Chicago
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 Director, Center for Pediatric Genomic Medicine Children’s Mercy

1:30 PM - 02:00 PM	“Death and Gene Therapy for Leukodystrophies”	Joshua Bonkowsky, MD, PhD Chief, Division of Pediatric Neurology Director, Primary Children’s Center for Personalized Medicine Bray Chair in Child Neurology Research Schmidt/Edgar Chair in Pediatric Neurology
2:00 PM - 02:30 PM	“Biomarkers in the Diagnosis and Management of Aicardi Goutieres Syndrome”	Adeline Vanderver, MD Jacob A. Kamens Endowed Chair in Neurological Disorders and Translational Neurotherapeutics Program Director, Leukodystrophy Center of Excellence Children’s Hospital of Philadelphia Professor of Neurology and Pediatrics Perelman School of Medicine at the University of Pennsylvania
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
3:15 PM - 03:45 PM	“Can We Improve Rehabilitation for Women with X-ALD?”	Jennifer Keller, MS, PT Kennedy Krieger Institute
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 Neurogenetics and Gene Therapy
4:15 PM - 04:30 PM	Closing	Geneviève Bernard, MD, MSc, FRCPc Full Professor, Departments of Neurology and Neurosurgery, Pediatrics, and Human Genetics, McGill University Pediatric Neurologist, Department of Specialized Medicine, Division of Medical Genetics, McGill University Health Center Scientist, Child Health and Human Development, RI MUHC Chair, ULF Medical and Scientific Advisory Board
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	