

Rare Disease Day

2024 Symposium

& CDG Scientific and Family Conference

Congenital Disorders of Glycosylation: Models and Therapies









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Rare Disease Day Scientific and Family Conference March 1-3, 2024, San Diego, CA

March 1-2

Keynote Presentations:

Richard Cummings, PhD, Harvard University. The Human Glycome Landscape and CDG Joni Rutter, PhD, Director, NCATS NIH. Accelerating Treatment and Cures for Rare Diseases

Invited Scientific Presentations

Eva Morava MD PhD, Mayo Clinic. Novel Therapies for CDG

Clara Van Kamebeek, PhD, Amsterdam University. From Models to Targets and Treatments Xiao-Dong Gao, PhD. Jiangnag University. In vitro Assays of ALG Glycosyltransferases Heather Flanagan-Steet PhD, Greenwood Genetic Center. Untangling Neurological CDGs Hudson Freeze PhD, Sanford Burnham Prebys. Fucose: More than a Therapeutic Sugar Tamas Kozicz MD PhD, Mayo Clinic. Neural Dysregulation in CDG: Insights and Therapies Christian Thiel, PhD, University of Heidelberg. Biotin Can Treat Most CDG Patients Silvia Radenkovic, PhD, Mayo Clinic. Tracer Metabolomics Leads to New Therapies Kathrin Meyer PhD, The Ohio State University, TBD

Lance Wells, PhD, University of Georgia. *O-linked CDG, Inside and Outside*Peter McWilliams, PhD, Glycomine Therapeutics. *Clinical Trials with Liposomal Man-1-P*Joe Contessa, MD, PhD, Yale University. *Can We Restore N-glycosylation?*Ryan Weiss PhD, University of Georgia. *Drug Discovery for Multiple Hereditary Exostosis (MHE)*Youngmok Lee PhD, University of Connecticut. *Novel Treatment Rescues Neutropenia in GSD-lb*Marjan Huizing PhD, NHGRI. *Therapies for Free Sialic Acid Storage Disease*Matthew Gentry PhD, University of Florida; *Metabolic Channeling of Sugars in Brain Metabolism*Melissa Schlemmer, Morgan Liddle, Ashley Linthicum, Lewis Franklin: Stories of Patients & Advocates

Added Feature

Doctor is In Session: Scientists and Clinicians Answer Patient/Family Questions

March 3: CDG CARE- Family Advocacy and Support Organization Program