



***For Immediate Press Release!!**

It is with great excitement that we share with our CDG Community patients, families, professionals and stakeholders that the National Institutes of Health has awarded a \$5 million, 5-year grant for the Frontiers in Congenital Disorders of Glycosylation Project!

This project, also referred to as the CDG Consortium Project, leverages cross-disciplinary, team-based clinical science to address decades of unresolved questions, increase clinical trial readiness, advance and share knowledge, develop treatments, and address current unmet patient needs.

“This is such great news for all of us. After decades of managing the best we could with our limited resources, CDG families will now get a chance for greater coordination, better access, and multi-level coordination. We are ready for the next big CDG Family journey. I’m so excited. Let’s get started!” says Hud Freeze, Ph.D. Professor of Human Genetics at Sanford Burnham Prebys Medical Discovery Institute

The CDG Consortium Project establishes a nation-wide network of 10 regional academic centers, the Sanford Burnham Prebys Medical Discovery Institute and the patient advocacy groups, CDG CARE and NGLY1.org who will work together to accomplish the following research goals:

- 1) To define the natural history of CDG, validate patient reported outcomes and share CDG knowledge;
- 2) Develop and validate new biochemical diagnostic techniques and therapeutic biomarkers to increase clinical trial readiness; and
- 3) Evaluate whether dietary treatments restore appropriate glycosylation to improve clinical symptoms and quality of life.

The Principal Investigator of the CDG Consortium Project is Eva Morava-Kozicz, M.D., Ph.D. Professor of Medical Genetics at the Mayo Clinic. The Mayo Clinic in Rochester, Minnesota will be the site with dedicated staff overseeing the Administrative Coordinator and Trial Coordinator positions.

Dr. Morava-Kozicz shares these words with the CDG Community, “In my career, I have focused on research on CDG but never had a chance to work so closely with the patients to study the disease, share the results and develop therapies hand in hand. I’m honored to be the recipient of this grant which is fully focused on improving the quality of life of CDG patients. I’m very excited to collaborate on improving diagnostics and care in addition to starting clinical trials and sharing knowledge with our 11 collaborative centers and our wonderful patients and families.”



Enrollment for the natural history study will begin in approximately six months, and enrollment into the clinical trials will begin next summer. All patients diagnosed with CDG are eligible for enrollment into this natural history trial, with the primary focus on CDG Type 1 diagnosis.

Specific clinical trials will focus on SLCA35A2-CDG, PGM1-CDG and NGLY1. Biomarker discovery will include all patients enrolled at the different CDG Consortium Project sites, and data will be shared with patients in the studies through a data Cloud. The Sanford Burnham Prebys Medical Discovery Institute, along with Children's Hospital of Philadelphia (CHOP) and Mayo Clinic will work on the diagnostic markers.

Enrollment will be continuous through the summer of 2024 at the Mayo Clinic and the additional participating academic medical centers listed below:

University of Minnesota Masonic Children's Hospital, Minneapolis, MN

Tulane University Medical Center, New Orleans, LA

Children's Hospital of Philadelphia (CHOP), Philadelphia, PA

National Institutes of Health, Bethesda, MD

Boston Children's Hospital, Boston, MA

UPMC Children's Hospital of Pittsburgh, Pittsburgh, PA

Seattle Children's Hospital, Seattle, WA

Children's Hospital Colorado, Denver, CO

Baylor College of Medicine, Houston, TX

Patient and family support are critical components in making this multi-year project successful! CDG CARE and NGLY1.org will be sharing contact information on timelines and how to enroll in this natural history study and clinical trials in the coming months.

We thank all of the families who helped our efforts in applying for this grant opportunity last Fall. If you are interested in receiving future emails about CDG research opportunities, please sign up to receive CDG CARE News Highlights and Events emails from our Homepage, or send an email directly to research@cdgcare.com.