Frontiers in Congenital Disorders of Glycosylation Consortium and CDG CARE

Call for Pilot and Feasibility Projects

Introduction

The Frontiers in Congenital Disorders of Glycosylation Consortium (FCDGC) and CDG CARE jointly request full proposals for funding during the 2023-2024 grant year. These awards are intended to support projects that will provide preliminary data for new, extramural grant submissions. The review criteria will emphasize innovation and the potential of the project, if successful, to have a significant impact on an important research problem. FCDGC funds will be available for one award with up to $50,000 in direct costs for each award. CDG CARE, an affiliated CDG Patient Advocacy Group, will also be reviewing application submissions to identify an additional research project that may receive an award of up to $50,000. The duration of the awards will be 12 months.

Background

Frontiers in Congenital Disorders of Glycosylation Consortium (FCDGC) is one of the 20 federally funded consortia to advance medical research on rare diseases by providing support for clinical studies and facilitating collaboration, study enrollment and data sharing.

Eva Morava-Kozicz, M.D., Ph.D., Clinical Genetics, is the Principal Investigator and Director of the Frontiers in Congenital Disorders of Glycosylation Consortium.

The purpose of the FCDGC program is to facilitate the development of pioneering research, clinical trials, training and outreach in the area of congenital disorders of glycosylation.

The grant (U54NS115198) is funded by the National Institute of Neurological Disorders and Stroke, coordinated via National Center for Advancing Translational Science and originates from the NIH Director's Common Fund.

As a collaborative partner of the FCDGC, CDG CARE is the Patient Advocacy Group representing CDGs and NGLY1 in the USA. CDG CARE is a 501(c)(3) organization whose mission is to promote awareness, provide education and support scientific research

to advance the diagnosis and treatment of all CDG. Research awards provided by CDG CARE originate from donor designated funds received through annual fundraising campaigns. Applications to be considered for separate funding by CDG CARE will emphasize clear potential for direct impact for CDG patients and families.

Program Goal

The FCDGC provides funds for projects that will exclusively focus on CDG related science and it is meant to produce preliminary data supporting areas of research that are either new in the CDG field or new to an established investigative team. We will especially focus on projects that have the potential for high-impact on CDG patient care. The scope of projects supported by this P&F award mechanism will include any step of the CDG care continuum, ranging from novel concepts in clinical management, developing novel diagnostics, as well as exploring pathomechanisms and new therapeutics (human studies) in CDG. FCDGC will value proposals that are in the high risk, high impact category. The consortium also aims to form new multidisciplinary collaborations that will enhance the integration of the techniques offered at the participating institutions (list below) that will benefit multiple investigators. Potential applicants are encouraged to explore basic, translational, and clinical research. Extending the collaborative nature of research projects with funds from other mechanisms is encouraged. The strengths of our consortium are extensive expertise in clinical management, expert laboratory science, excellent clinical laboratories, clinical trials with novel therapeutics, rich source of natural history, an ever-growing biobank, and access to patient advocacy groups, all focused on improving the diagnosis and clinical management of patients with CDGs.

Non-Responsive Proposals

FCDGC will only consider proposals based on human studies. However, CDG CARE will consider animal and cell line model-based proposals for funding. This triaging will be automatically performed by the proposal review committee.

Participating Centers in FCDGC

The below centers participate in the FCDGC consortium. Proposals that develop collaborations with these centers are encouraged, but not necessary. These centers also have expertise in clinical management for CDGs as well as clinical laboratory and research laboratory expertise. This expertise can be leveraged for the proposals. Potential investigators are encouraged to speak with the center's principal investigator while developing collaborative proposals.

1. Mayo Clinic, Dr. Morava-Kozicz; Morava-Kozicz.Eva@mayo.edu
2. CDG CARE; Patient Advocacy Group; Ms. Andrea Miller; research@cdgcare.org
3. Baylor College of Medicine; Dr. Scaglia; fscaglia@bcm.edu
4. Boston Children's Hospital; Dr. Berry; Gerard.Berry@childrens.harvard.edu
5. Children's Hospital of Colorado; Dr. Larson; Austin.Larson@childrenscolorado.org
6. Children's Hospital of Philadelphia; Dr. He; HeM@email.chop.edu

7. Children's Hospital of Pittsburgh at the University of Pittsburgh Medical Center; Dr. Vockley; vockleyg@upmc.edu
8. National Human Genome Institute; Dr. Gahl; gahlw@mail.nih.gov
9. Sanford Burnham Prebys Medical Discovery Institute; Dr. Freeze; hudson@sbpdiscovery.org
10. Seattle Children's Hospital; Dr. Lam; Christina.Lam@seattlechildrens.org
11. Tulane University Medical School: Dr. Andersson; handers@tulane.edu
12. University of Alabama; Dr. Ananth; aananth@uabmc.edu
13. University of Minnesota Masonic Children's Hospital; Dr. Sarafoglou; saraf010@umn.edu
14. Emory University – Dr. Neira; juanita.neira.fresneda@emory.edu
15. University of North Carolina Chapel Hill – Dr. Jalazo; elizabeth.jalazo@unchealth.unc.edu
16. G. Rodolico Polyclinic University Hospital, Catania– Dr. Barone; rbarone@unict.it
17. UZ Leuven – Dr. Witters; peter.witters@uzleuven.edu
18. Lund University – Dr. Eklund; erik.eklund@med.lu.se
19. Porto University – Dr. Quelhas; dulcequelhas.cgm@chporto.min-saude.pt
20. University of Alberta – Dr. Andrews; saadet@ualberta.ca
21. Rady Children's Hospital – Dr. Wigby; kwithers@health.ucsd.edu

Eligibility

All basic, translational, or clinical investigators located at institutions within the United States who are eligible to apply as a Principal Investigator for NIH grants. Applications from investigators and/or institutions outside of the USA will be accepted and reviewed for funding by CDG CARE. Early-stage investigators and investigators who are new to the field of CDG are especially encouraged to apply. Investigators who have extensive research programs in CDGs are generally ineligible for funding (i.e. R01 style grants focused on CDG).

Application Funding Source Selection

This RFA offers two different funding sources: FCDGC and CDG CARE. Both organizations will perform an initial review to select applications that are of interest to CDG CARE. All applications go through the same review as outlined below. Applications that are of interest to CDG CARE will go through an additional review by CDG CARE scientific committee. No effort on the part of applicant is needed to request a funding source.

Funding

- Up to $50,000 in direct costs for each award. Indirect costs will not be covered.
- Eligible expenses include costs for clinical data collection, sample collection, laboratory data collection, data analysis and animal care. Laboratory data collection is to be performed with FCDGC labs and the expense is covered.
- Investigator effort will not be supported.
- Project duration of one year.

- NIH application guidelines will be followed.

Review procedure

Content experts will form the review panel. Recommendations for funding will be forwarded to the FCDGC Executive Committee, who will make final approvals for funding. A separate review by CDG CARE scientific committee will also be conducted for applications that are of interest to CDG CARE. Applications will be scored using the current NIH Scoring System.

Review criteria will include the following:

- Alignment with goals of program.
- Scientific merit and innovation.
- Likelihood to support novel research questions that leverage the resources of the FCDGC.
- Likelihood of future extramural funding.
- Feasibility within time and budget proposed.
- Qualifications and scientific environment of the investigators.
- For laboratory science-based projects, priority will be given to projects which have samples ready for analysis.

Application Information

Applications are due no later than 01/03/2023. Earliest anticipated start date is 03/15/2023.

Applications must include the following components and use PHS 398 continuation format page unless otherwise specified.

A. Cover letter including project title, principal investigator, co-investigators, and a paragraph summary of the project.

B. Specific aims — One page outlining the goals of your project.

C. Research plan — Background, hypothesis, prior work summary, experimental plan, timeline and extramural funding as well as justification for the use of the FCGDC services and resources (includes clinical data collection/storage, sample collection/storage, laboratory services, data analysis services, etc.). A two page limit is enforced for this section.

D. References cited.

E. Biographical sketches — Please use the PHS 398 biographical sketch format for each PI and Co-Investigator.

F. Budget and Justification — Include a budget (budget limit of $50,000 direct per application) and justification. Please use the PHS 398 Detailed Budget for Initial Budget Period format page.

G. Human Subjects and Clinical Trials — The FCDGC will require that all applicants for P&F awards follow the NIH criteria to define and recognize proposals involving human subjects and human clinical trials. We will require any proposal involving human subjects to follow all instructions for the PHS Human Subjects and Clinical Trials Information form in the SF424 (R&R) Application guide. Applications that are not compliant with NIH human subject and clinical trial policy will not be considered for funding.

H. Inclusion of Women, Children and Minorities — The FCDGC will require that applicants for P&F awards carefully consider the inclusion of women, children, and minorities in clinical studies using relevant NIH guidelines. However, we recognize that given the rare nature of the disease, small size of the awards and their hypothesis generating nature, large diverse sample sizes are not realistic for all human studies.

Please bundle all application documents into one PDF file. Submit via email to FCDGC@mayo.edu. Please indicate "Frontiers in Congenital Disorders of Glycosylation Consortium Application" in the subject line.

Please contact Eva Morava-Kozicz MD, PhD (Morava-Kozicz.Eva@mayo.edu) to discuss clinical management, natural history and clinical trial expertise for the proposal. Please contact Surendra Dasari, PhD (dasari.surendra@mayo.edu) to discuss clinical laboratory services. Please contact Hudson Freeze, PhD (hudson@sbpdiscovery.org) or Tamas Kozicz, MD, PhD (Kozicz.Tamas@mayo.edu) to discuss research laboratory services.

Reporting and Tracking of Impact

Progress reports will be required at the end of the funding period and yearly for two years after the end of the funding period. Information tracked will include the following:

- Grants applied for and funded.
- Publications.
- Impact on career development.
- Impact on clinical practice.
- Invention disclosures or other commercialization activities resulting.

Publications

The NIH Public Access Policy requires that all publications resulting from NIH funding be uploaded to PubMed Central. The following link will guide awardees through the process of uploading publications.

Awardees must cite the Frontiers in Congenital Disorders of Glycosylation Consortium grant (U54NS115198) or CDG CARE, whichever is appropriate, as a funding source in any resulting publications.

Questions

If you have any questions about the grants or the application process, please contact Dr. Surendra Dasari (dasari.surendra@mayo.edu) or Dr. Eva Morava-Kozicz (Morava-Kozicz.Eva@mayo.edu) or Ms. Andrea Miller (research@cdgcare.org).