



November 1, 2017

Call for Expressions of Interest

Research Grants for CBS Deficient Homocystinuria

HCU Network America and HCU Network Australia have recently established a research grant program and a Scientific Advisory Board (SAB) to identify and advise on research priorities for classical homocystinuria (CBS deficiency), with a view to expand to include other homocystinurias over time. Based on an SAB meeting held in September 2017, this call for Expressions of Interest (EOI) is being initiated to identify a small number of potential researchers whose programs will be considered for grants and invited to submit a Grant Application.

Eligible Applicants

Eligible applicants must have a Ph.D., M.D., or equivalent degrees and currently hold a full-time position at any level (post-doctoral, research scientist, assistant/associate/full professor, etc.) at an established academic/research institution. Applicants will be considered from the United States, Australia and internationally.

Key Priorities

Key priorities for EOIs to be considered include:

- Technologies to improve newborn screening (and potentially also aid in ongoing monitoring or screening of undiagnosed older individuals); and
- Potential new therapies to treat homocystinuria caused by cystathionine beta synthase deficiency (with the ability to treat elevated homocysteine caused by methylation disorders and cobalamin deficiencies an added benefit).

Awards are expected to be up to \$40,000; however exceptional applications may be funded at a higher amount if the budget request is justified. The award requires that funds not be used for the support of institutional indirect or overhead costs.

Applicants are encouraged to use the funding to support innovative seed or

pilot projects to produce preliminary data that would facilitate more substantial funding from federal agencies, foundations or corporations.

Expression of Interest

Researchers are asked to submit an Expression of Interest (EOI) Application, not exceeding 2 pages, (single-spaced with 0.5" margins using no less than 11 point Arial fonts), and including high level description of the proposed project, potential budget range and any conflict of interests with SAB members.

EOI required details include:

- Project Title
- Principal Investigator Name
- Abstract/Summary of Project – no more than 350 words
- Hypothesis to be tested
- Significance of proposed research
- Project timeline (assuming project start July 1, 2018)
- Project Budget (budget range is acceptable for EOI)

Please submit Expressions of Interest by January 15, 2018 to Margie McGlynn at margie@themcglynns.net and Tara Morrison at tara@hcunetworkaustralia.org.au

EOIs will be reviewed by the SAB, in consultation with HCU Network America and HCU Network Australia, and applicants will be notified by **March 1, 2018** if they are invited to submit a Grant Application.

Potential conflicts

An affiliation with one or more of the SAB members is considered a conflict of interest and must be stated in the covering letter. SAB members are noted below.

For **additional information** on the research strategy and application process for this grant program please visit:

HCUNetworkAustralia.org.au/research-hcu/
or
hcunetworkamerica.org/research-grants/

Scientific Advisory Board members:

Viktor Kožich, (Chair of SAB); MD, PhD, Professor of Medical Genetics, Department of Pediatrics and Adolescent Medicine, General University Hospital in Prague and Charles University-First Faculty of Medicine, Prague, Czech Republic

Matthias R. Baumgartner, Prof. Dr. med., head of Division for Metabolic Diseases und Medical Director of the Swiss Newborn Screening Program, Kinderspital, Zürich, Switzerland

Henk Blom, Ph. D

Kimberly Chapman, MD, Ph.D., attending physician in Genetics and Metabolism, Children's National: Assistant Professor of Pediatrics and Integrated Systems Biology, George Washington University, U.S.

Jan Krause, Ph. D, Professor Pediatrics / Cell and Developmental Biology, University at Colorado U.S.

Warren Kruger, Ph. D, Full professor, Cancer Biology program at Fox Chase Cancer Center, Philadelphia, PA, U.S.

Andrew Morris, MD, Consultant and Senior Lecturer in Paediatric Metabolic Medicine. Willink Metabolic Unit, Central Manchester University Hospital, UK

Professor Bridget Wilcken, AM, MD, FRACP, FRCPA (hon), FHGSA, emeritus consultant at the Children's Hospital, Westmead, Sydney; part-time metabolic physician, Sydney Children's Hospital, Australia

Patient Representatives:

Tara Morrison, BA LLB, Director and Chair, HCU Network Australia

Margaret McGlynn, R. Ph., Ph. D. (hon), President of Board, HCU Network America