



March 21, 2024

Call for Research Proposals

The National PKU Alliance (NPKUA) works to improve the lives of individuals with phenylketonuria (PKU) and to pursue better treatments. The NPKUA Fund is a campaign of the NPKUA to advance the science of PKU by funding the most promising research that will lead to enhanced management, new therapeutic approaches and ultimately a cure to improve the lives of individuals with PKU. The goals of the Fund are to:

- Support new treatments and/or management strategies to bring to market within the next five years.
- Make substantial grants to projects each year that focus on a new management strategy, new drug development, new treatment(s) and/or a cure. A variety of management and treatment solutions need to exist for PKU patients because it is not a one-size-fits-all disease.
- Support development of new clinical outcome assessment tools to improve treatment decisions and accelerate drug development to optimize the day-to-day management of PKU.
- Fund personnel placements in labs and/or clinics where additional support will speed up the drug development process.
- Fund the most critical projects that have the potential to raise the quality of life of a PKU patient on a day-to-day basis so they can live the best life possible.

The NPKUA is seeking proposals that will help us meet the above strategic goals, promote and encourage investigators into PKU-related research, close existing gaps in the knowledge and science of PKU, and further support in existing PKU-related projects. Potential areas of interest for this grant cycle include research on:

- Somatic comorbidities, including obesity
- Biomarker research, including neurotransmitters
- Preclinical models
- Managing psychological and neurocognitive comorbidities

Eligibility of Applicants

Eligible applicants must have an advanced (i.e. PhD, MD, SW, GC, RD) or equivalent degree(s) and hold a full-time position at an established clinical/academic/research



institution. Both United States and international academic institutions will be considered. Investigators from centers with a history of research in metabolic diseases are strongly encouraged to apply. The average award is \$50,000; however exceptional applications may be funded at higher amounts if the budget request is justified. Initial grants are awarded for one year with the potential of renewal based on progress made during the first year. In addition, the NPKUA supports some strategic projects on an on-going basis.

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.

Application Guidelines

Applicants are invited to submit a **full proposal** (single-spaced with 0.5" margins using no less than 11-point Arial fonts). Full proposals should include **the following in order** and be submitted in one electronic document in the order listed below and be no more than 15 pages in total:

- Project Title
- PI Name
- Abstract/Summary of Project – no more than 350 words
- Introduction
- Hypothesis to be tested
- Significance of proposed research
- Specific Aims including primary and secondary objectives and endpoints
- Methods and procedures
 - Cells, animals, or patients selection/handling
 - Research methods/tools
 - Materials used
 - Data collection methods and analyses
 - Population to assess
- Proposed results & potential pitfalls with some solutions including potential benefits and harms
- Project timeline
- Project Budget to include an institutional fiscal officer's signature and itemized justification (no more than 1 page)



- Project Personnel (no more than 3-page consistent with an NIH type short bio-sketch).

Applicants without a faculty appointment (e.g. post-doctoral level, clinician) must also submit a letter of support from their project mentor or employment supervisor.

The award requires that funds not be used for the support of institutional indirect or overhead costs.

Each application is reviewed by the NPKUA Scientific Advisory Board and the NPKUA Scientific Advisory Council and decisions are made based upon medical and scientific merit that are in line with the goals of this program. The NPKUA Executive Board reviews all decisions made by the Advisory Board and Council and makes the final decision based on available resources and strategic research priorities.

Timeline:

Full proposals should be received no later than June 17, 2024. Award notices will be given by September 2024 with funding to begin in October 2024. Payments may be given in a lump sum or milestone payments pending on the nature of the grant awarded.

Proposals will be funded by NPKUA for one year and the submission of a progress report will be required within 6 months after the grant has been awarded, along with goals for renewal funding consideration. A final report will be due 90 days after the end of the funding period. NPKUA also expects grant recipients to publish the findings of their research, whether or not the results the study are favorable, published in a scientific journal or presented at a scientific conference, so that others may benefit from their work. Any publication or presentation related to the study should be shared with the NPKUA for our reference, and acknowledgement of NPKUA funding should be included. IRB or IACUC approval must be obtained prior to distribution of NPKUA funds.

Please submit proposals and all materials in the order listed and in one document electronically to: **Elaina Jurecki, NPKUA Director of Research Development,** elaina@npkua.org.