



THE FOUNDATION FIGHTING BLINDNESS

INSTRUCTIONS FOR THE PREPARATION OF PRPH2 AND ASSOCIATED RETINAL DISEASES (PARD) SCIENCE AWARD APPLICATIONS

This document provides instructions for the preparation of PRPH2 and Associated Retinal Diseases (PARD) Science Award applications to the Foundation Fighting Blindness (FFB) via the online application portal. These instructions follow the sections of the online application portal.

Applications for the PRPH2 and Associated Retinal Diseases (PARD) Science Awards may be submitted ONLY by those individuals who have been invited by the FFB.

GENERAL INFORMATION AND KEY DATES

KEY DATES:

Application Receipt Date: **Applications must be received by December 20, 2023**

Review of Applications: February 2024

Earliest Anticipated Award Date: April 2024

ELIGIBLE INSTITUTIONS AND PRINCIPAL INVESTIGATORS

Principal Investigators must hold an M.D., D.M.D., D.V.M., D.O., O.D., Ph.D. or recognized foreign equivalent and occupy a faculty position or equivalent position at a college, university, medical school, or other public or private research institution/facility. **Application is not limited to the U.S. or U.S. citizens. Applicants who are not U.S. citizens and reside and work outside the U.S. may apply.** Individuals from underrepresented racial, ethnic and gender groups, as well as individuals with disabilities, are always encouraged to apply.

FFB CONTACT FOR INQUIRIES

Direct inquiries regarding the application and review process and required application components and forms to:

Tel: 410-423-0583, 1-800-683-5555

Email: grants@FightingBlindness.org

DURATION OF AWARD AND LIMITATIONS ON BUDGET REQUESTS AND ALLOWABLE COSTS

1. Support may be requested for a period of **one to three (3) years**.
2. **Total** budget requests may not exceed a total of **\$500,000**. ***Projects that are shorter in duration or do not require the full budget are also suitable.***
3. Purchase of capital equipment is generally not supported by the FFB. Capital equipment is defined as permanent or semi-permanent apparatus, devices or systems costing more than \$5,000 per item or system. Applicants must obtain prior approval from FFB to submit an application proposing to purchase equipment. Contact the FFB Senior Director, Grants and Awards Program or the FFB Chief Scientific Officer for prior approval. In addition, if approval is granted to submit a request, all such equipment purchase requests must be well justified in the budget section of the application and in the description of proposed project.
4. Indirect and other costs: FFB does not pay any indirect or overhead charges, nor does the Foundation provide support for construction or renovation costs.

ANIMAL, RECOMBINANT DNA AND HUMAN SUBJECT ASSURANCES

FFB, like the National Institutes of Health (NIH), uses the "Just in Time" concept. Applicants may defer, until after completion of peer review and just prior to funding: certification of Institutional Review Board (IRB) and Institutional Biosafety Committee (IBC) approval of the application's proposed use of human subjects and proposed use of recombinant DNA; verification of Institutional Animal Care and Use Committee (IACUC) approval of the proposed use of live vertebrate animals; Health Insurance Portability and Accountability Act (HIPAA) compliance; and, evidence of compliance with the requirement for education in the protection of human research participants.

Evidence of IRB, IACUC, and IBC approval must be documented by submission of a signed FFB Institutional Agreement Form (IAF) at the time of award. If approvals are pending at the time of award, the FFB funding cannot be expended for research involving human subjects, recombinant DNA, and live vertebrate animals until the signed FFB IAF is submitted to document that the appropriate approvals have been obtained.

REVIEW CRITERIA

The Foundation will make awards for meritorious research proposals that advance our understanding of the biology and pathophysiology of PRPH2 in the retina. To address gaps in knowledge, proposals that focus on the following topics are strongly encouraged:

- Fundamental PRPH2 biology and biochemistry, including, but not limited to:
 - PRPH2 protein-protein interactions
 - The differential role(s) of PRPH2 in rod and cone photoreceptors
- Development of high throughput and/or saturation assays to determine the functional consequences of PRPH2 variants on protein structure and/or function and to assign pathogenicity.
- Creation and characterization of precise, accurate animal models, including, but not limited to:
 - Systematic natural history studies of mouse disease models on standardized genetic backgrounds
 - Generation and characterization of gene-edited large animal disease models
- Studies demonstrating proof-of-concept for therapeutic approaches, particularly mutation-agnostic approaches.
- Improved understanding of disease heterogeneity, through understanding of genotype-phenotype relationships or identification of genetic and/or environmental modifiers

In their written critiques, reviewers will use the criteria listed below to assess the likelihood that the proposed research will have a substantial impact on the pursuit of this goal. Each of these criteria will be addressed and considered in assigning the overall score, weighing them as appropriate for each application.

1. **Significance.**

- Does the study address an important problem? How is the study

unique?

- If the aims of the application are achieved, how will scientific knowledge or clinical practice be advanced?
- What will be the effect of the study on the concepts, methods, technologies, treatments, services, or preventive interventions that drive the field?
- What is the feasibility/plan to take the proposed studies/treatments to the clinic?

2. **Approach.**

- Are the conceptual or clinical framework, design, methods, and analyses adequately developed, well integrated, well reasoned, well supported by preliminary data, and appropriate to the aims of the project?
- Does the applicant acknowledge potential problem areas and consider alternative tactics?
- Are the annual timelines realistic and feasible for achieving the specific aims of the proposed project?

3. **Innovation.**

- Is the proposed research original and innovative? **For example**, does the proposed research project challenge existing paradigms or clinical practice; address an innovative hypothesis or critical barrier to progress in the field?
- Does the proposed research project develop or employ novel concepts, methodologies, tools, or technologies for this area?

4. **Investigators.**

- Are the investigators appropriately trained and well suited to carry out this research?
- Is the research proposed appropriate for the experience level of the Principal Investigator and other researchers?
- Does the investigative team bring complementary and integrated expertise to the project?

5. **Environment.**

- Does the scientific environment in which the research will be done contribute to the probability of success?
- Does the proposed study benefit from unique features of the scientific environment, or subject populations, or employ useful collaborative arrangements? Is there evidence of institutional support?
- Are plans for collaborations with other FFB-supported investigators adequate with respect to sharing methodologies/technologies, animal models, patient assessment tools and results, and both positive and

negative findings?

APPLICATION FORMATTING INSTRUCTIONS

- Use an **Arial typeface and a font size of 11 points or larger**. (A Symbol font may be used to insert Greek letters or special characters; the font size requirement still applies.)
- Use a 1” margin
- Type density, including characters and spaces, must not exceed **15** characters per inch.
- Type may be no more than **six lines** per inch.
- All page limits specified refer to **single-spaced** format using the above formatting requirements.
 - **ABSTRACT**: up to 1-single-spaced page
 - **SPECIFIC AIMS/RATIONALE**: up to 2 single-spaced pages
 - **PRELIMINARY/SUPPORTING DATA**: up to 2 single-spaced pages
 - **DETAILED PROJECT DESCRIPTION**: up to 9 single-spaced pages
 - **BUDGET and BUDGET JUSTIFICATION**: up to 2 pages

APPLICATION SUBMISSION INSTRUCTIONS

All applications must be submitted online. Attachments are required and must be submitted through the application portal.

NOTE: The complete application must be SUBMITTED by December 20, 2023.

Online submission

First create an account on the site’s homepage by selecting “Applicant Registration-start here” underneath the FFB logo. If you have previously created an account, this step is not necessary.

You may log out and return to your in-progress application as many times as you wish until it has been submitted. In order to be considered for the award, your online application must be complete and in SUBMITTED status no later than **DECEMBER 20, 2023 by 11:59 p.m. EST** .

A. How to enter information

1. You may begin completing the application at any section. To begin, choose a section of the application from the left menu or click "Continue" at the bottom of the screen.
2. Text boxes will hold only a limited amount of text. Where longer answers are permitted, the number of available characters will be indicated. **Before submitting, we suggest you print and examine a hard copy of your application to be certain your responses are complete and accurate.**
3. The information you provide will be saved exactly as entered. Therefore, fill out the form carefully, paying attention to spelling, case (**do not use all caps**), punctuation, et cetera, and give special consideration when entering your contact information.
4. Begin typing all answers at the extreme left hand side of the response area or box; do not leave a space or indent at the beginning of your answer.

B. How to SAVE and SUBMIT your data

1. You must **SAVE each time you leave a screen**. If you do not click on SAVE or SAVE & CONTINUE, anything entered since you last hit SAVE on that screen will be lost (any work from a previous session will be retained, but any new entries will be lost). The SAVE and SAVE & CONTINUE buttons are at the bottom of your screen.
2. You may work on your application over as many sessions as you wish, and the status of your application will be **IN PROGRESS** until you submit it. Once you are satisfied that your application is complete, you must go to the "Submit Application" screen and select **SUBMIT APPLICATION**.
3. When you have completed your application, we strongly suggest that you print and read it before submitting, to be sure there are no further revisions you wish to make. **If for some reason you need to make changes after your application is submitted, email blindness@onlineapplicationportal.com.**
4. Your application status must appear as SUBMITTED **by DECEMBER 20, 2023 11:59 p.m. EST** in order for your application to be considered. Information on your application status may be found on the Online Application Portal home page.

APPLICATION COMPONENTS

- **APPLICATION FACE PAGE**

The application Face Page must be signed by the Principal Investigator and the responsible institutional individual. Prior to submitting the application, print the face page, obtain the appropriate signatures and upload as instructed on the application portal Face Page Upload page.

- **KEY PERSONNEL**

Each PRPH2 and Associated Retinal Diseases (PARAD) Science Awards Application must be directed by a **single Principal Investigator** who is responsible for the conduct and management of the project. Co-Investigators are allowed and must be identified.

1. Add all key scientific and technical personnel involved in the proposed project. Designate researchers who are co-investigators.
2. Provide current and pending sources of ALL research support. For each source (federal, private or commercial) provide: title, grant number, percent effort, funding amount, and budget period. This information should include total support for all current and proposed projects.
3. Provide abbreviated CVs for all key personnel (NIH biosketch is acceptable), listing **ONLY RELEVANT** publications from the last three years and representative publications prior to that. **DO NOT** include Abstracts.

- **ABSTRACT** (limit: 1 single-spaced page, see Application Formatting Instructions)

Provide an abstract of the proposed research project, written **in lay terms** for a non-scientific audience. The abstract should contain non-confidential material that can be posted on FFB's web site if the application is funded.

Include the following:

1. The research question(s) to be investigated.
2. The significance of the proposed project in terms of accelerating the advancement of therapeutic and preventive approaches into the clinic, and how the proposed research directly supports the mission of FFB.

3. A **brief lay** description of **all specific aims**, including experimental approach (es), and a listing of all diseases/patient populations to be studied.
4. The expected accomplishments and outcomes for each specific aim.

- **SPECIFIC AIMS AND RATIONALE** (limit: **1-2** single-spaced pages, see Application Formatting Instructions)

1. Describe the overall goal(s) and rationale for the proposed project. Numerically list the specific aims and describe the anticipated results to be achieved in each year of the project. Note that successful applicants will be required to submit Annual Progress Reports that detail accomplishments for each of the specific aim identified in the application.
2. Describe the potential clinical value of the proposed research in terms of developing therapeutic and preventive interventions for inherited orphan retinal degenerative diseases and dry age-related macular degeneration, including the feasibility of applying the anticipated results to the development of new or improved interventions.
3. For clinical research projects, identify the disease(s) and patient population(s) to be studied.
4. Briefly discuss how you envision FFB funding of this proposed research to promote, supplement, or complement future support from the NIH and/or other funding agencies/organizations.

- **PRELIMINARY/SUPPORTING DATA** (limit: **2** single-spaced pages, see Application Formatting Instructions)

Describe existing experimental data or prior clinical research that support(s) the soundness and feasibility of the proposed experiments. Include evidence of *in vitro* and/or *in vivo* experiments, if applicable, that demonstrate the relevance of the proposed experiments for advancing therapeutic and preventive interventions.

- **DETAILED PROJECT DESCRIPTION** (limit: **9** single-spaced pages, see Application Formatting Instructions)

1. **Experimental Plan and Methods:** (limit: 5 single-spaced pages, see Application Formatting Instructions)

For each specific aim, describe the experimental design, procedures and methods to be used. **The level of detail in a NIH investigator-initiated research project Award application is not required by the FFB.**

However, applicants must include sufficient information so that reviewers can understand the proposed experiment, its soundness, feasibility and importance for advancing the study of inherited orphan retinal degenerative diseases and **dry** age-related macular degeneration.

Specific Instructions for PRPH2 and Associated Retinal Diseases (PARD) Science Awards Applications Proposing Research Using Human Samples

Applications proposing **research using human samples** must provide the information delineated below.

NOTE: If IRB approval is not required in order to conduct the proposed clinical study using human samples, then such projects are considered non-clinical and applicants are not required to provide the information/materials listed below.

Clinical Research Requirements:

A. *Study Description:* A description of the proposed clinical study, including: (a) hypothesis and study objectives; (b) study population(s) and relevance of the proposed study to clinical disease/patient outcome; (c) study design, methodologies, and the scientific rationale, including supporting data from completed basic, preclinical and clinical research, and the feasibility and appropriateness of applying such supporting data to the design and execution of the proposed clinical study; (d) statistical analysis plan; and, (f) plan for receipt and storage of human samples.

B. *Human Samples:* Documentation of the ability to acquire human samples prospectively or retrospectively, including obtaining samples from planned, ongoing or completed clinical studies/trials sponsored by any source. This should include written agreements between the applicant institution, the clinical trial sponsor, and the Investigational New Drug (IND) or Investigational

Device Exemption (IDE) sponsor, if not one of the above, for the conduct of the proposed study.

C. *Informed Consent*: A copy of the approved or proposed informed consent form to be used for collection of patient samples **(include in Appendix Materials, Key Publication Section; does not count against 9-page proposal limit)**.

2. **Milestones and Timelines**: Delineate key milestones and timelines for the proposed research project. Milestones are intended to define the specific stages and/or steps involved in accomplishing the stated aims, and are to incorporate timelines for the initiation, execution and completion of each specific stage or step. The number of types of milestones will vary depending on the goals and intended outcomes of the proposed research project. This information may be shown in a Gantt chart.
3. **Collaborative Plans**: Include plans for collaboration with other investigators to share research materials, methodologies/technologies, animal models, patient assessment tools and results, and both positive and negative findings.
4. **Future Relevance**: If the aims of the application are achieved, **describe how scientific knowledge or clinical practice will be advanced**. Include a brief discussion on the anticipated effect of the study on the concepts, methods, technologies, treatments, services, or preventive interventions that drive the field of research on inherited orphan retinal degenerative diseases and dry age-related macular degeneration.
5. **References**: Provide a list of references and abstracts for: (a) publications relevant to the data cited to support the science for the proposed research; and, (b) up to **five** pertinent reprints representing the applicant's research. Copies of the reprints can be uploaded on the **KEY PUBLICATION** page. This information does **not** count against the page limits for the Detailed Project Description.

- **BUDGET** (limit: 1-2 pages)

For each year of support requested, provide a detailed, itemized budget and budget justification for each of the categories listed below.

1. **Personnel:** Listed by name with percent effort, salary, and fringe benefits requested. **Salary Support for the Principal Investigator of up to twenty (20) percent of the total annual budget is permitted. There is no salary cap.**
2. **Supplies:** (Itemized by category, e.g. glassware, molecular biology reagents, not by individual items within the category).
3. **Patient Costs:** (Itemized)
4. **Animal Costs** (Itemized).
5. **Travel Costs (limits):**
 - **\$2,000** per annum (**U.S., Canada**)
 - **\$2,500** per annum (**Europe**)
 - **\$3,000** per annum (**e.g. South America, Australasia, India, Japan, China**)
6. **Other Costs:** (Itemized).

Applicants MUST use the standard FFB Budget format provided as an **Excel template** in the PRPH2 and Associated Retinal Diseases (PARD) Science Award Application Package which can be downloaded within the application portal [below]. If you are unable to download the files, contact the FFB to obtain the form. Applicants are to submit the proposed budget in U.S. dollars.

Note: The FFB no longer allow an annual ‘cost of living’ increase.

The budget WILL NOT convert to PDF and therefore WILL NOT be visible to you in the copy of your completed application. FFB will receive the excel file of the submitted budget.

- **LETTERS OF COLLABORATION**

Upload, if appropriate, letters of collaboration for all proposed collaborators (Word or PDF format). There is no limit on letters of collaboration.

- **KEY PUBLICATIONS**

Upload up to five pertinent reprints representing the applicant’s research individually in PDF format. Upload each reprint as a separate PDF document. Do not combine the reprints into one document.

- **PRINT & SUBMIT APPLICATION**

Before submitting, we suggest you examine the final copy of your application to be certain your responses are complete and accurate. The budget will not convert to PDF and therefore will not be visible to you in the final copy. Unlike the Face Page sections of the application a physical signature is not required when submitting your full application. Follow the instructions listed on the Print & Submit Application page to “sign” and submit your fully reviewed and completed application.