

Pachyonychia Congenita Research Grant Guidelines

MISSION

PC Project is dedicated to finding effective treatments and a cure for Pachyonychia Congenita (PC), a painful, debilitating skin disorder, through helping and connecting patients, empowering research, and partnering with industry.

VISION

A day when PC sufferers will live without excruciating pain, isolation, and embarrassment.

INTRODUCTION AND OVERVIEW

Patients with Pachyonychia Congenita (PC) suffer daily with debilitating pain, typically from painful calluses on the soles of the feet or inflamed cysts on their skin. PC is a rare genetic skin disorder caused by spontaneous or inherited, autosomal dominant mutations affecting the coding sequence of one of five keratin genes, *KRT6A*, *KRT6B*, *KRT6C*, *KRT16* or *KRT17*.

A vital part of PC Project's mission is facilitating and funding research. PC Project is committed to funding promising investigations that can lead to approved therapies and ultimately a cure for PC. Since its founding in 2003, the PC Project has supported an open grant application system and funding for researchers interested in PC on a case-by-case basis. With the goal of maximizing the impact of resources available and accelerating research relevant to PC, the PC Project Board of Directors met in October 2022 and elected to establish a formal Pachyonychia Congenita Research Awards and Grants Program.

Funding awards given by PC Project will be based on the "PC Research Agenda", a published document that is focused on patient needs and was authored by researchers and clinicians through multidisciplinary collaboration. Accordingly, PC Project's grants will support investigators interested in conducting innovative PC research. Discovering new avenues for therapies (of which there are currently none), and one day, a cure for PC are key aims of these awards.

RESEARCH FUNDING PRIORITIES

PC Project seeks to fund innovative research with these goals:

1. Fundamental research that will increase understanding of the mechanisms underlying the various symptoms of PC.
2. Translating these discoveries into diagnostic and therapeutic advances for PC.
3. Leading to improved quality of life for individuals with PC.

ELIGIBILITY AND REQUIREMENTS

Investigators at all levels, from clinical and post-doctoral fellows to established investigators, are eligible to receive awards. Non-U.S. investigators are eligible, and the research funded by PC Project can be conducted at non-U.S. institutions. Investigators must be members of an accredited academic institution. All research projects involving human subjects must receive IRB approval (see below). Research funds from PC Project will only be paid directly to the Investigator's institution (grantee institution). Specific information about allowable expenses and other relevant guidelines is provided below.

FEDERAL REGULATIONS; IRB and IACUC APPROVAL

Investigators are required to comply with all applicable federal, state, location, or regional regulations that pertain to their research. All research involving human subjects or animals must have proper regulatory approval before any work takes place. Applicants must attest to IRB/IACUC approval status in the online application, and awardees must agree to provide a copy of IRB/IACUC approval as a condition of funding. Proof of ongoing approval is necessary on a yearly basis. Applicants are strongly encouraged to obtain IRB approval before applying to begin research within the required six-month window (see "Award Period" above).

APPLICATION REVIEW

PC Project's Research Committee will review and rank award applications. The PC Research Committee will evaluate each application using criteria that includes scientific merit of the proposal, investigator track record, institutional environment, and impact of the proposal on the mission of PC Project. Final approval and funding of applications will be made by PC Project's Board of Trustees.

CONDITIONS OF AWARDS

Notification: The principal investigator and the grantee institution will be informed in writing about the duration and amount of the grant awarded.

Award Period: Projects are expected to begin within six-months of the application due date. Special requests to extend this time-period must be submitted in writing to info@pachyonychia.org and will be evaluated on a case-by- case basis.

Payment: To be made in one or multiple payment sums depending on the grant amount and timeline. The first payment will be made within approximately 30 days of receipt of the signed PC Project Research Award Contract or upon request of the grantee.

USES OF FUNDS

Funds obtained from PC Project may be used only for the specific use(s) set forth in the grant proposal and contract. Funds not used during the grant period for the specific use(s) set forth in the grant contract must be returned to PC Project at the end of the grant term unless an exception has been granted in writing by the PC Project. Requested funds such as for researchers, consumables, and facility access changes must be clearly detailed and costed in the proposal.

The following expenses are not allowed:

- a. salaries, benefits and/or stipends for persons not specifically identified in the grant budget;

- b. institution administrative and facilities costs, except for up to 5% of the grant which can be specified for indirect costs, if needed;
- c. general fundraising expenses;
- d. journal subscriptions, advertisements, tuition fees, professional society dues;
- e. travel and related expenses (except to the extent travel pertaining to study participants is included in the approved grant budget), meals or entertainment;
- f. lobbying.

PROGRESS REPORTS

Interim Progress Report: A report of scientific progress on the research project is due within thirty (30) days of the mid-point of the grant award period. Failure to meet this requirement will result in withholding of the 2nd installment of grant funds until the report is submitted and may result in early termination of the award. (If applicable and it's a multi-payment grant.)

Final Research Report: The final progress report will be a scientific report including a list of titles and publications of papers supported all or in part by this research award. The final report shall be submitted to PC Project within sixty (60) days of the end of the grant. The undersigned acknowledges that failure to submit a final report on time will result in automatic elimination of consideration for subsequent awards.

ACCOUNTING REQUIREMENTS

Final Financial Report: A full reconciliation of all expenses made for a research project is due within sixty (60) days of the completion/termination of the grant. Reconciliations should be emailed to info@pachyonychia.org by the financial officer of the grantee's institution. Unexpended funds remaining at the end of the grant must be returned to PC Project. PC Project is not responsible for over-expenditures of grants. Unallowable costs charged to the grant must be reimbursed to PC Project.

Rebudgeting is not allowed without prior approval from PC Project.

PUBLICATIONS AND REPRINTS

PC Project grants are awarded with the expectation that the most important results of the funded research will be presented at scientific meetings and ultimately published in peer-reviewed journals. The responsibility for both lies with the principal investigator.

When a manuscript funded wholly or in part by a PC Project grant is accepted for publication, a copy of the accepted version, with the journal Editor's acceptance notification should be sent electronically to info@pachyonychia.org.

If a press release from the institution is being prepared for distribution, PC Project would like to participate in the process. Please notify PC Project at info@pachyonychia.org.

PC Project should be acknowledged in all dissemination materials (i.e. publications, scientific presentations, press releases, etc.) related to research supported in full or in part by the Foundation. The following acknowledgment, or its equivalent, should be used: "This work was supported (in part) by a grant from Pachyonychia Congenita Project."

LIABILITY POLICY:

PC Project is not responsible for any claim, judgment, award, settlement, negligence, or malpractice arising from a research project or investigation funded or related to an award provided by PC Project. The awardee and institution acknowledge responsibility for the conduct of research or investigations related to the award resulting from any act or omission on the part of the awardee and/or institution, their employees, agents, or representatives to the extent allowed by applicable law, and release PC Project from all responsibility.

INTELLECTUAL PROPERTY OWNERSHIP:

The Institution shall retain ownership of any intellectual property created using PC Project grant funds. However, PC Project reserves a non-exclusive royalty free license to use any intellectual property created by Institution using PC Project Grant funds for non-commercial purposes.

POLICY ON SCIENTIFIC MISCONDUCT AND FRAUD:

Any institution that receives funds from PC Project must have written policy guidelines on conflict of interest and research integrity including scientific misconduct and fraud. It is the responsibility of the institution and the grantee/awardee to inform PC Project immediately, in writing, of any institutional investigation into the conduct of an investigator whose work is supported by PC Project, and to keep PC Project informed in a timely manner of the progress and outcome of such investigation. A finding by the institutional investigation that the investigator was guilty of research fraud or misconduct represents sufficient grounds to terminate PC Project's support of a project.

The following materials must be submitted with your application:

APPLICATION CONTENTS

- A. Title page and Abstract
- B. Layperson's statement (3-4 sentences max; use non-expert terminology).
- C. Budget and timeline (one page max)
- D. Applicant's biographical sketch (NIH format) (four pages max)
- E. Research proposal (five pages max)

A. TITLE PAGE AND ABSTRACT

Include the project title, principal investigator (PI) name and affiliations, and the addresses of the PI and the official to whom correspondence should be addressed.

B. LAYPERSON'S STATEMENT

In four sentences or less, describe the goals the project you are proposing and its significance for patients with pachyonychia congenita using language that a person who is not trained in or does not have a detailed knowledge of this subject would understand. This statement should have a layman's title, relate the aims of the project and its significance to the understanding or treatment of PC. The layperson's statement will be used for a variety of PC Project communications including its website, newsletter, press releases, award presentations, and others.

C. BUDGET

A budget must be submitted. A concise narrative of how you propose to allocate funds must be included. PC Project may award funds based on the original budget or make conditional awards

dependent upon requested adjustments to the original budget. Funds may only be used for the purposes outlined in the original budget and research proposal. Any significant deviation from the original budget and/or project must be approved by PC Project with a written justification.

Departmental and Indirect Costs: Award funds are to be used only for the designated recipient and project for which the application was submitted. Five percent of the award funds may be used for indirect costs of the institution or department.

D. APPLICANT'S BIOGRAPHICAL SKETCH

A biosketch must be submitted. Please use the format used by the National Institutes of Health in the United States, or the equivalent.

E. RESEARCH PROPOSAL:

This section includes a description of the research proposal in sufficient detail for adequate evaluation by the Scientific Review Committee. Applicants should make every effort to be succinct and use figures or tables to summarize their plans. **The research proposal, including items 1-5 below, cannot exceed 5 (five) pages, including figures and tables but excluding references. Applications exceeding this page limit will be returned.** Font size can be no smaller than Arial 11 point (or the equivalent character density).

Suggested Format:

1. Specific Hypothesis and Aims
Describe the hypothesis that is to be tested and the specific aims.
2. Background and Significance
Describe why the research is important and what has already been done in the field. Evaluate existing knowledge in the field, and specifically identify the possible contribution your investigation may make.
3. Preliminary Studies
Describe in detail what has already been accomplished on the project to support the hypothesis and aims.
4. Experimental Design
Describe in detail the experimental design, the methodology, tools, and models to be used, and the manner in which the data will be analyzed. Do not include details of established laboratory procedures.
5. Future Plans
Describe how the successful achievement of the proposed specific aims will lead to a competitive application for a larger grant.
6. Environment and Resources
Describe in detail your organizational structure regarding resources and institutional commitment to pachyonychia congenita clinical services and/or research. Include where the work will be performed and sources of any supplemental funding supporting the project.

7. Literature Cited

Include all literature cited in your proposal.

PC Project will award the following types of grants:

Two Catalyst Research Grants – up to \$50,000

Designed to support talented early-career scientists on the path toward becoming the next generation of PC thought leaders by supporting hypothesis-driven research projects.

One PC Project Champion Research Grant – up to \$100,000

Encourages established researchers to pursue research on ongoing or emerging challenges in PC or bring their expertise to the field of PC.

Grant Application Portal Opens: May 10, 2023

Submission Deadline: August 31, 2023

Award Announcements: November 1, 2023