Cystic Fibrosis Research Translation Center
Pilot and Feasibility Program

I. OVERVIEW

The Pilot and Feasibility (P&F) program, directed by Dr. Pradeep Singh, manages pilot studies funded by the Cystic Fibrosis Research and Translation Center (CFRTC) at the Seattle Children's, the University of Washington and its affiliated institutions. These include the University of Washington Medical Center, the Fred Hutchinson Cancer Center, Seattle Children's Hospital/Research Institute, the Benaroya Research Institute, Seattle Biomedical Research Institute, and other affiliate institutions that operate under a sub-contractual relationship with Seattle Children's and the University of Washington. Funding for these pilot projects are issued under an NIH P30 DK089507 awarded to Seattle Children's Hospital and the UW, and Co-directed by Drs. Bonnie Ramsey and Pete Greenberg.

The objective of the P&F program is to serve the CFRTC mission of advancing CF research to improve the health of patients with this devastating disease. The P&F program will contribute to this mission by recruiting young investigators to CF-related research, recruiting established investigators who have not previously worked in CF research, and to enable accomplished CF investigators means to pursue novel and high risk ideas that represent departures from their established work.

II. GUIDELINES

Eligibility and Related Guidelines

The P&F program is particularly directed at new investigators and established investigators new to CF research. Established CF investigators pursuing high impact/high risk projects or projects that are a significant departure from their usual work are also eligible for support under the CF Research and Translation Core Center P&F program. This Program should be integrated into the overall research goals of the Center and **Pilot Projects should make use of the resources provided by the CFRTC P30 Cores.** P&F programs may also be structured to provide support for establishing interdisciplinary collaborations and to help forge new partnerships between basic scientists and clinical researchers. In general, NIDDK expects CF Research and Translation Centers to solicit investigators at affiliated hospitals or institutions to participate in the Center P&F program. While the distribution of P&F funds to be used in each award category is ultimately at the discretion of the Center P&F committee, it is expected that the Center P&F program will, where possible, place particular emphasis on funding innovative clinical and translational research projects.

Funding and research plan: A Pilot and Feasibility study provides modest research support for a limited time (one to two years) to enable eligible investigators to explore the feasibility of a concept related to the mission of the Center and generate sufficient data to pursue the project through other funding mechanisms. The pilot and feasibility studies are intended to:

(1) provide initial support for new investigators;

(2) allow exploration of possible innovative new leads or new directions for established investigators and

(3) stimulate investigators from other areas to lend their expertise to research in this area. Pilot and feasibility study support is not intended for large projects by established investigators which would otherwise be submitted as separate research grant applications. Pilot and feasibility funds are also not intended to support or supplement ongoing funded research of an established investigator.

A proposed pilot and feasibility study should present a testable hypothesis and clearly delineate the question being asked, detail the procedures to be followed, and discuss how the data will be analyzed. It must be on a topic related to the objectives of the Core Center. Projects should be focused, since funding for these studies is modest and is limited to two years or less. Any one investigator is eligible only once for this support, unless the additional proposed pilot and feasibility study constitutes a real departure from his/her ongoing research.

Pilot and Feasibility projects proposing clinical studies are encouraged. The National Center for Advancing Translational Sciences (NCATS) supports Clinical and Translational Science Awards (CTSA) nationwide, which provide services and resources to enhance clinical research (http://www.ctsaweb.org/). Research Centers supported by the NIDDK and other NIH Institutes and Centers are encouraged to collaborate with CTSAs to avoid duplication of effort and enhance utilization of services and resources.

***Investigator Eligibility*:** Investigators eligible for pilot and feasibility funding generally fall into three categories:

(1) new investigators without current or past NIH research support (R01, P01) as a principal investigator (current or past support from other sources should have been modest);

(2) established investigators with no previous work in CF who wish to apply their expertise to a problem in this area; and

(3) established investigators who propose testing innovative ideas that represent clear departure from ongoing research interests. It is expected that the majority of the investigators will fall into the first category. **All eligible investigators, however, must have faculty appointments and be independent investigators.** Postdoctoral fellows or their equivalents are not eligible.

Each pilot and feasibility study proposal should state clearly the justification for eligibility of the investigator under one of the above three criteria.

III. APPLICATION INFORMATION (see PHS 398 Guidelines and Forms for more details):

Cover Letter/email (include the following)

- List the names and contact information of 3 possible external reviewers for your proposed application.
- List the names of individuals (e.g. competitors) who should not review the application and why.
- Statement of applicant eligibility indicating under which of the 3 criteria listed above the applicant is applying.
- Description of which of the CFRTC Core(s) (Clinical, Microbiology, Genomics, Host Response) will support the pilot and feasibility project.

Face Page

Institutional signatures from SCH or UW are not required for submission. Awarded pilots will be included in the overall parent grants for those institutions and sign-off obtained during annual progress report submissions. Other affiliate institutions should provide an LOI or signed face page upon submission or at the latest prior to award date. Earliest funding start date is 6/1/18.

Description/Summary and Relevance to Cystic Fibrosis (Abstract) (30 lines)

Detailed Budget

Limit $75,000 direct costs per year for up to 2 years. Earliest proposed funding start date 6/1/18.

Budget for Entire Proposed Project and Justification

Checklist

F&A rates for Seattle Children's Hospital and University of Washington are set based the effective rate at the time of the last competing cycle for the parent award (6/1/2015). If any of the rates have decreased since that agreement date, the lower F&A rate shall be applied. As of November 2017, those rates are as follows:

Seattle Children's Hospital

- Clinical: 50% (Rate Agreement Date 11/16/15)
- Bench: 88.3% (Rate Agreement Date: 12/16/16)

University of Washington

- On Campus: 54.5% (Rate Agreement Date: 7/21/17))
- South Lake Union: 74% (Rate Agreement Date: 7/21/17)
- Off Campus: 26% (Rate Agreement Date: 7/21/17)

Biographical Sketch(es)

Other Support

Resources/Facilities

Specific Aims (1 page) - Clearly state the aims of the Pilot and Feasibility Study.

Research Strategy (5 pages)

a. Significance/Preliminary Studies
b. Innovation
c. Approach

Literature Cited

Protections for Human Subjects

For research that involves human subjects but does not involve one of the six categories of research that are exempt under 45 CFR Part 46, please provide justification for involvement of human subjects and the proposed protections from research risk relating to their participation according to the following five review criteria: 1) risk to subjects, 2) adequacy of protection against risks, 3) potential benefits to the subjects and others, 4) importance of the knowledge to be gained, and 5) data and safety monitoring for clinical trials.

For research that involves human subjects and meets the criteria for one or more of the six categories of research that are exempt under 45 CFR Part 46, please provide: 1) the justification for the exemption, 2) human subjects involvement and characteristics, and 3) sources of materials. For additional information on review of the Human Subjects section, please refer to the Guidelines for the Review of Human Subjects.

Refer to Supplemental Instructions Part II of the PHS 398: Supplemental Instructions for Preparing the Protection of Human Subjects Section of the Research Plan and Human Subjects Research Policy if the proposed research will involve human subjects.

PHS Inclusion/Planned Enrollment Report

If this application involves the Inclusion of Women and Minorities, complete the PHS Inclusion Enrollment Report for each protocol; see Part II Supplemental Instructions for Preparing the Human Subjects Section of the Research Plan and Human Subjects Research Policy, Section 4.3.

Prior Approval of clinical trials/greater than minimal risk to human subjects

Awardee-selected projects that involve clinical trials or studies involving greater than minimal risk to human subjects require approval by NIH prior to initiation.

- The awardee institution will provide NIH with written study protocols that address risks and protections for human subjects in accordance with NIH's Instructions for Preparing the Human Subjects Section of the Research Plan.
- The awardee institution will provide NIH with specific plans for data and safety monitoring, and will notify the IRB and NIH of serious adverse events and unanticipated problems, consistent with NIH DSMP policies.

Vertebrate Animals

If Vertebrate Animals are involved in the project, address each of the criteria listed below.

- Provide a concise, complete description of the animals and proposed procedures.
- The responses to the criteria below must be well-integrated with the other sections. There should be sufficient detail in the responses for peer reviewers and NIH staff to evaluate. Additional details, if any, may be included in the Research Strategy.
- Identify all project/performance or collaborating site(s) and describe activities of proposed research with vertebrate animals in those sites.
- Address the following criteria as succinctly as possible. An incomplete application will not be considered for review. It will be considered incomplete if the following criteria are not addressed.

If plans for the use of animals have not been finalized, explain when and how animals are expected to be used.

If an award is made, the grantee must provide:

- detailed information on the criteria below; and
- verification of IACUC approval.

These must be submitted to the NIH awarding office by the parent grant institution prior to the involvement of animals.

An applicable Animal Welfare Assurance will be required if the grantee institution does not have one (see Part III, Section 2.2 Vertebrate Animals for more information).

The criteria are as follows:

1. Description of Procedures. Provide a concise description of the proposed procedures to be used that involve vertebrate animals in the work outlined in the "Research Strategy" section. Identify the species, strains, ages, sex, and total numbers of animals by species, to be used in the proposed work. If dogs or cats are proposed provide the source of the animals.
2. Justifications: Provide justification that the species are appropriate for the proposed research. Explain why the research goals cannot be accomplished using an alternative model (e.g.computational, human, invertebrate, in vitro).
3. Minimization of Pain and Distress: Describe the interventions including analgesia, anesthesia, sedation, palliative care and humane endpoints to minimize discomfort, distress, pain, and injury.
4. Euthanasia: State whether the method of euthanasia is consistent with the recommendations oft he American Veterinary Medical Association (AVMA) Guidelines for the Euthanasia of Animals. If not, describe the method and provide a scientific justification.

For additional information, see https://grants.nih.gov/grants/olaw/VASchecklist.pdf.

Do not use the vertebrate animal section to circumvent the page limits of the Research Strategy..

Letters of Support: Include any letters of support for the Pilot and Feasibility Project by the appropriate consultant/collaborator.

Resource Sharing Plan: Individuals are required to comply with the instructions for the Resource Sharing Plans (Data Sharing Plan, Sharing Model Organisms, and Genome Wide Association Studies (GWAS)) as provided in the SF424 (R&R) Application Guide.

Appendix: Do not use the Appendix to circumvent page limits. Follow all instructions for the Appendix as described in the SF424 (R&R) Application Guide.