Cystic Fibrosis Research Development Program
Pilot and Feasibility Study Application
Policies and Guidelines

POLICIES AND GUIDELINES FOR PILOT AND FEASIBILITY STUDIES

Funds from the RDP may be used for young investigators or established investigators from diverse basic or clinical science disciplines who may apply their expertise to the problems of CF. Funding of these studies is intended to provide modest support enabling investigators to obtain sufficient preliminary data to successfully compete for longer-term, more substantial funding from agencies such as NIH. Funding of **individual basic science pilot and feasibility studies should not exceed $50,000 per year nor extend beyond two years**.

Translational pilot and feasibility projects are projects that seek to: apply basic scientific knowledge to a clinical problem, acquire basic knowledge from clinical specimens, or examine the feasibility of a therapeutic approach in an appropriate animal model or with appropriate clinical specimens. **Translational pilot and feasibility projects may receive funding for up to $70,000 per year** for up to two years. Translational pilot and feasibility projects may not subject human volunteers to experimental medicines, devices or techniques; obtain clinical specimens prospectively unless such specimens are collected as a part of routine patient care; or make use of Health Insurance Portability and Accountability Act-protected confidential patient information.

General examples of pilot and feasibility studies include:

- Studies proposed by young investigators interested in pursuing a career in CF research. Such investigators may not yet have obtained individual grant support.
- Studies proposed by established investigators who have experience in an area related to CF and wish to test their abilities to contribute new knowledge on CF. In some cases, this may represent applying techniques, methods, approaches, or theories from other fields to the problems of CF.
- Studies proposed by established CF investigators who wish to develop and/or test a new approach that is not an extension of their ongoing research.

Examples of relevant topics include but are not exclusive to:

a. Chronic lung inflammation
b. Lung microbiology and chronic infection.
c. Bacterial-host cell interactions
d. Bacterial physiology and antibiotic resistance as related to chronic airway infections
e. CF model systems
f. Lung transplantation

Pilot and feasibility studies **DO NOT** support or supplement an investigator's ongoing research. In addition, projects with sufficient preliminary data should not seek RDP Pilot and Feasibility support, but rather should apply to other agencies (e.g. NIH) for continued support.

Pilot and Feasibility projects addressing key questions related to understanding F508del CFTR processing defects or CF airway microbiome, physiology and inflammation remain priorities for CFF. Applicants are also encouraged to consider how pharmacological restoration of CFTR activity impacts the airway milieu including resident pathogens. Other newly emerging research opportunities to be considered include: molecular characterization of CFTR mutations other than F508del, examination of potential approaches to restore CFTR function regardless of mutation, overcoming barriers to gene and oligonucleotide transfer, non-embryonic stem cell therapy, and identification/optimization of tools for personalized medicine.

INSTRUCTIONS FOR COMPLETING THE APPLICATION

COVER LETTER/E-MAIL

Please submit a cover letter/e-mail including the names, institutions and e-mail address of 3 possible external reviewers for your project.

FACE PAGE

Please complete all requested information. Institutional sign-off is NOT required during the submission process. Awarded Pilot Projects and PI's will be linked to the University of Washington eGC1 and sign-off process for the non-competing renewal for the parent grant under Dr. Pradeep Singh (SINGH15R0).

The dates of the Proposed Project and the total Proposed Budget should be for the full 2 year period of the request. The parent budget fiscal year runs from October 1st – September 30th. In most cases, funding is awarded using this fiscal year cycle.

Applications may be submitted prior to IRB and/or IACUC approval. Funding, however, will be withheld until final IRB and/or IACUC approvals have been obtained.

ABSTRACT AND SUMMARY OF RELEVANCE

Scientific Abstract

Provide a statement of **no more than 250 words** explaining the subject of the research proposal and its relationship to CF. This statement will be used for the scientific community.

Summary of Relevance to CFF mission

All applications are reviewed and scored not only on scientific merit but also on relevance to CFF's mission.
The mission of the Cystic Fibrosis Foundation is to cure cystic fibrosis and to provide all people with the disease the opportunity to lead full, productive lives by funding research and drug development, promoting individualized treatment, and ensuring access to high- quality, specialized care.

Provide a statement of **no more than 250 words** summarizing the relevance of the proposed research to the health and well being of CF patients, for a scientific audience who may or may not have a background in the subspecialty of the proposed research.

BUDGET

Please complete the detailed budget request and budget justification for **Year 1.** If Year 2 funding will be significantly different than Year 1, please include a second detailed budget and justification for **Year 2** as well.

Budget requests for basic science Pilot and Feasibility studies should not exceed $50,000/year.
Translational Pilot and Feasibility studies may request up to $70,000/year. Translational pilot and feasibility projects are projects that seek to: apply basic scientific knowledge to a clinical problem, acquire basic knowledge from clinical specimens, or examine the feasibility of a therapeutic approach in an appropriate animal model or with appropriate clinical specimens.
No indirect costs are allowed on RDP grants.

DETAILED BUDGET – DIRECT COSTS

Personnel - List the names and positions of all professional and non-professional personnel involved in the project, whether or not salaries are requested. Indicate the percent of time or effort per week on the project for professional personnel; indicate the hours per week for each non-professional. For each individual, list dollar amounts separately for salary and fringe benefits. In accordance with **National Institutes of Health (NIH) policy, the institutional base salary of an individual should not exceed the current federal salary cap of $187,000.** Fringe benefits may be requested if they are treated consistently by the applicant institution as a direct cost to all sponsors. The percentage of salary requested cannot exceed the percent effort for each professional and non-professional personnel.

Consultant Costs - Give the name and institutional affiliation of any consultant who has agreed to serve in this capacity, including statisticians and physicians in connection with patient care if they are not listed under personnel. Under budget justification, briefly describe services to be performed, the number of days, rate of compensation, per diem and any other associated costs.

Equipment - List all items of equipment requested and the cost of each item. If funds are requested to purchase equipment that is equivalent to items listed under Facilities Available, justify the duplication. Justify any item of equipment for which the need may not be obvious.

Supplies - Itemize supplies, such as glassware, chemicals, animals, etc., in separate categories and give the estimated cost of each category. If animals are involved, state the number, unit purchase cost, and unit care cost.

Travel - Describe the purpose of any travel. Funds for travel outside the North American continent are not permitted. Also note that requests for travel funds should be limited to $1,250.00.

Other Expenses - Itemize other expenses by major categories, such as duplication costs, publication costs, computer charges, equipment maintenance, etc. Justify all items.

Subcontracts –Detailed budgets for each subcontract must be provided. Negotiations of subcontracts are between the applicant institution and the subcontractor.

BUDGET JUSTIFICATION

Use this page to describe the nature of costs listed in the "Detailed Budget." Costs should be described in terms of major categories, such as Personnel, Consultant Costs, Equipment, etc.

BIOGRAPHICAL SKETCH

Include an NIH Biographical Sketch for all key project personnel, beginning with the Principal Investigator. (CFF defines "key project personnel" as any individual with an advanced degree that will play an instrumental role in the accomplishment of the research project.) Do not exceed **five (5) pages** per person.

OTHER SUPPORT

Provide a list of all current and pending other support for each key personnel. There is no page limitation for Other Support. NIH Other Support pages are accepted.

FACILITIES AVAILABLE

Describe the facilities and equipment available at the applicant's organization that will be used for this project. Describe their pertinent capabilities, proximity, and anticipated extent of use. If facilities or equipment at a consultant's or collaborative site will be used, they should be identified and clearly described. Use continuation pages, if necessary.

Describe **facilities** in terms of relevant areas, such as laboratory, clinical, animal, computer, office, etc. Provide any **additional information** about the environment, including any support services available that will be utilized for this project.

RESEARCH PLAN

(7 pages maximum)

The research plan for **Pilot and Feasibility** study applications is limited in length to seven (7) single-sided pages, including the Literature Cited. Applications exceeding this page limit will not be reviewed.

If the Pilot and Feasibility project is dependent upon or related to any Core included in the RDP, there must be a description of the relationship.

A. Hypothesis and Specific Aims. State concisely and realistically the intent of the proposed research and the hypothesis to be tested. Do not exceed one page. **When preparing the specific aims, keep in mind the mission of the Cystic Fibrosis Foundation.**

B. Background and Significance. Briefly describe the background of the present proposal. Critically evaluate existing knowledge and specifically identify the gaps that the project is intended to fill. Concisely state the importance and rationale of this research by relating the specific aims to longer-term objectives. This section should also show the potential importance of the proposed work to CF. In addition, the applicant should describe the relationship of the proposed work to his/her long-term career goals. Preference will be given to those applicants who have expressed an interest in a long-term career in CF-related research.

C. Preliminary Results. If applicable, provide a detailed discussion of any preliminary results.

D. Experimental Design and Methods. Provide a detailed discussion of the experimental design and methods to be used to accomplish the specific aims. Describe the protocols, including methods for new techniques, and explain potential advantages over existing methodologies. Discuss the data expected to be obtained and the means by which data will be analyzed and interpreted. If clinical studies are involved, provide details of the methods for patient selection and care. Discuss potential difficulties and/or limitations of the proposed procedures and alternative approaches to achieve aims. Point out any procedures, situations or materials that may be hazardous to personnel or patients and the precautions to be exercised. Since Research Grants are reviewed by CFF's Research and Research Training Committee those applications that include methodologies requiring sampling of materials from human subjects will only be considered under this mechanism if the sampling method constitutes minimal patient risk (e.g., venipuncture) and patient samples or data are anonymous. The level of risk and measures taken to assure patient anonymity to the PI and other professional personnel, unless the PI or other professional personnel are care providers, should be described.

E. Consultant Arrangements. If the proposed project includes consultant arrangements and/or collaboration with other individuals outside the applicant's group, describe the working relationships and support this description by letter(s) of intent signed by collaborating individual(s). If clinical material required by this grant is to be furnished by other individuals, include a statement from these individuals agreeing to their participation and precautions taken to ensure anonymity of patients.

F. Literature Cited. References should be numbered in the sequence that they appear in the text and listed at the end of the Research Plan. Each citation must include the names of authors, the name of the journal or book, volume number, page number and year of publication (titles are optional).

ORGANIZATION ASSURANCES & CERTIFICATIONS (to be completed upon notification of award)
Research Involving Human Subjects
CFF policy pertaining to the protection of individuals as research subjects requires that for each proposal submitted, the applicant institution certify in writing that an Institutional Review Board (IRB) has reviewed and approved the procedures for the use of human subjects, or human organs, tissues and body fluids, in the proposed research, in accordance with Department of Health and Human Services policies. This certification **must** be received prior to activation of any grant. If the approval is pending, give the date when it is expected. The approved certification should be submitted as soon as it is available.

Research Involving Recombinant DNA
All research involving recombinant deoxyribonucleic acid (DNA) techniques and human gene transfer supported by CFF must meet the requirements contained in the document *NIH Guidelines for Research Involving Recombinant DNA Molecules*. This publication and announcements of modifications and changes to the *NIH Guidelines* may be accessed at http://oba.od.nih.gov/rdna/nih_guidelines_oba.html. The purpose of these guidelines is to specify practices for the construction and handling of: (i) recombinant deoxyribonucleic acid (DNA) molecules, and (ii) organisms and viruses containing recombinant DNA molecules. As defined by those guidelines, recombinant DNA molecules are

either: (i) molecules that are constructed outside living cells by joining natural or synthetic DNA segments to DNA molecules that can replicate in a living cell; or (ii) molecules that result from the replication of those described in (i) above.

Many types of studies involving recombinant DNA are exempt from the *NIH Guidelines* while others are prohibited. The applicant organization is required to establish and implement policies that provide for the safe conduct of the research in full conformity with the *NIH Guidelines*. This responsibility includes establishing an Institutional Biosafety Committee to review all recombinant DNA research to be conducted at or sponsored by the applicant organization, and to approve those projects it finds are in conformity with the Guidelines.

CFF policy pertaining to recombinant DNA research requires that the applicant institution certify in writing that an institutional committee has reviewed and approved the procedures involving recombinant DNA in accordance with the *NIH Guidelines*. Applicants that do not have institutional committee approval must submit a recombinant DNA application to the applicant institution BEFORE the CFF application deadline. Certifications need not accompany the application, but all required certifications should be available upon request by CFF.

Research Involving Animals
Grant applications submitted to CFF involving the use of animals must meet the guidelines of the National Institutes of Health, U.S. Public Health Service, which require that all proposed studies be reviewed and approved by an Institutional Animal Care and Use Committee (IACUC). In addition, CFF grantee institutions and laboratories must be accredited by the American Association for Accreditation of Laboratory Animal Care (AAALAC) and/or have other verifiable assurances that the institution and laboratory meet appropriate standards. **Applicants that do not have institutional committee approval must submit an IACUC application to the applicant institution BEFORE the funding begins.** Certifications need not accompany the application. Applicants must be able to provide copies of all required certifications upon request.

Additional Materials
- Up to four (4) reprints of the applicant's work relating to the general area of research in the grant proposal may be uploaded in a PDF format.
- Letters of reference, support, and collaboration.
- Other materials pertinent to the grant proposal, not already described.

Keep in mind that extensive appendix material may not be reviewed. Please upload only the most relevant documentation.