GUIDELINES FOR PILOT & FEASIBILITY AWARD LETTERS OF INTENT

The Penn Gene Therapy Program CFF RDP requires that investigators who seek support for Pilot & Feasibility Award applications submit, in advance, a BRIEF DESCRIPTION of the research topic. The deadline for submitting Letters of Intent (LOI) is March 22nd. Letters of Intent (LOI) must be submitted as one complete PDF via email to moniquek@mail.med.upenn.edu - by 5:00pm (EDT) on March 22nd. The LOI guidelines can be found at http://www.med.upenn.edu/gtp/PilotApplicationGuidelines. Late submissions will not be accepted. The Committee reviews all LOIs electronically; therefore anything not submitted electronically will not be reviewed.

We have initiated this early review due to the high number of grant applications typically received and due to reductions in the CFF’s medical/scientific budget as a result of the economy. Moreover, many grant applications duplicate or overlap previously funded topics, are unrealistic in scope, or are inappropriate for the goals of research currently of interest to CFF (see summary below). By soliciting LOIs, we hope to avoid unnecessary time spent by both investigators preparing grants and reviewers examining these applications. Funding priority will be placed on those projects proposing to better understand the mechanisms behind disease pathophysiology and to develop strategies to prevent or treat it. Applicants will be notified by April 5th whether or not their LOI has been accepted for a full grant submission. If accepted, applications will be due by Monday May 3rd. Pilot & Feasibility applications must originate from independent investigators. In addition, the projects should focus on basic science or translational research.

The LOI is a brief description of the research project (maximum of 2 pages, not including references) and should include:

1. Project title;
2. Statement of hypothesis;
3. Goals of the research; and
4. Brief study design - must clearly state cell types or animal models to be used, aims to be addressed, and proposed methodology.

Note: Pilot & Feasibility Basic Science applications may receive funding of up to $40,000/year for up to two years and Translational applications may receive funding up to $80,000/year. No indirect costs are allowed on CFF RDP grants/pilots.

LOIs should be typed in Arial 11. Biosketches of key personnel will be required. The Gene Therapy Program's Executive Review Committee will review letters of intent and notify applicants as to the suitability of the study. Please contact Monique Molloy at moniquek@mail.med.upenn.edu if you have any questions regarding the program.
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Goals of Research Currently of Interest to CFF

Background

The majority of morbidity and mortality associated with cystic fibrosis (CF) today is due to lung disease. Within the endobronchial space mechanical, innate and acquired defenses work in a cooperative manner to maintain a “sterile” environment. In CF, one or more of these defenses are compromised and inhaled or aspirated pathogens are able to establish a chronic infection. However, cystic fibrosis is unique in that only a small subset of pathogens have been linked to disease progression and the infection remains, for the most part, compartmentalized. Containment of infection within the endobronchial space is highly likely due to exuberant inflammation. Unfortunately, the neutrophil dominant inflammatory response also causes tissue destruction compromising organ level function. Both the host and pathogen demonstrate adaptation as the initial infection evolves into an indolent, chronic infection punctuated by acute exacerbations. Based upon a series of meetings and an examination of currently funded work, the Cystic Fibrosis Foundation has identified several areas of research focus that are presently underrepresented within its portfolio; we have narrowed down this list to areas of specific interest to our Penn RDP. Investigators are encouraged to consider these areas when crafting a Letter of Intent.

Areas of current interest to the Penn CFF RDP:

- Gene therapy for CF;
- Mechanism(s) of improved airway mucociliary clearance by osmotic agents;
- Quantity and quality of airway submucosal gland secretions in response to physiologic stimuli and whether these are compromised in CF;
- Relationship between killing and clearance (mechanical and phagocytic) of inhaled bacteria and the impact upon inflammatory signaling;
- Determination of what selective processes, whether they be of the host and/or pathogen, favor chronic infection by particular strains of Pseudomonas aeruginosa;
- Rapid, minimally invasive means of detecting mucoid conversion of P. aeruginosa; and
- Development of more informative in vitro and in vivo models of chronic airways infection and inflammation.