Therapeutics Development Awards
Component I and II Awards

Letter of Intent (LOI) and Full Application

POLICIES AND GUIDELINES

Published: October 30, 2019
LOI & Full Application: Rolling submissions.
Deadline for current year funding: October 31
I. ABOUT THE CYSTIC FIBROSIS FOUNDATION

The mission of the Cystic Fibrosis Foundation (CFF) is to cure cystic fibrosis (CF) 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.

To achieve this mission, various types of grants and awards are offered to support meritorious research in CF.

II. THERAPEUTICS DEVELOPMENT AWARD (TDA) PROGRAM OVERVIEW

In an effort to stimulate development of new pharmaceutical products for CF patients, CFF developed the “Therapeutics Development Award” Program. The purpose of this program is to provide funds to companies that will develop commercial products to benefit individuals with CF. Component I and II TDAs are structured as a matching award program, funds will be awarded only if they are matched by the recipient.

With pressure on biotechnology companies to assure a return on investment to their shareholders, funds to initiate new exploratory research projects are limited. Funding currently available to companies is typically committed to products in the late stage of development. There has been a recent explosion in knowledge about CF; and a goal of the TDA award program is to provide funds that will spur companies to explore the feasibility of new therapeutic approaches to CF care.

The widespread scope of this program is meant to assure new product candidates are adequately explored. This funding mechanism is intended to support activities ranging from pre-clinical testing through clinical examinations of initial safety and efficacy. Once safety and proof of concept is demonstrated, CFF expects TDA awardees to obtain other financial support necessary to conduct pivotal Phase III clinical trials necessary for regulatory approval.

Two-Part Program

CFF generally requires that investigators and/or companies who seek support from the Foundation for TDA applications submit a Letter of Intent (LOI) in advance of a full funding application. Applicants may be able to by-pass the LOI with prior approval from the CFF Grants and Contracts Office.

Applicants may apply for funding based on the phase of the proposed research, as follows:
Component I – Discovery and/or Early Preclinical Development Phase: The objective of this phase may be to either apply platform technology to the discovery of compounds that may ultimately benefit people with CF or establish the technical merit and feasibility of a new therapeutic intervention. For discovery efforts, qualifying activities include assay development, screening, secondary assay analysis, medicinal chemistry and preliminary compound testing for pharmacokinetics and bioavailability, as well as specificity and determination of mechanism of action.

When a lead compound has already been identified, funding may be used for animal studies, toxicology, pharmacokinetics, and studies determining a potential new agent’s delivery. Funding is for up to US $300,000 per year for up to two (2) years of support ($600,000 total).

Component II – Late Preclinical and/or Clinical Phase: The objective of this phase is to provide support for the continuation of pre-clinical studies and clinical assessment of new interventions, including safety and dose determination studies in CF patients. Funding is generally not provided for chemistry manufacturing and controls (CMC) related activities or studies in healthy human subjects. Total funding levels are usually in the range of $3-$5M split over 2 or more years.

If applying for combined Component I and II funding over several years, the costs for each component are not limited to the individual component amounts, as long as the total CFF budget does not exceed the $3-5M range.

Funding Approach:

While not required, TDA funding components are designed to be applied for and funded sequentially. An application for Component I will examine the scientific potential of new products whereas applications for Component II studies will involve support for the continuation of Component I developments and the initiation of patient clinical studies. While applicants can apply for both components in the same application, no funding for Component II will be considered until the feasibility of Component I efforts has been successfully generated. However, applicants do not have to receive Component I funding prior to applying for Component II, as long as feasibility has been determined.

- Award payout is typically milestone-based and will be issued after CFF approval of accomplishment of each milestone (with third party supporting documentation as needed). If a TDA contributes to successful regulatory marketing approval, CFF will receive reimbursement plus a multiple and/or a percentage of net sales. Terms are negotiated prior to finalizing the award. If a program is unsuccessful, no payback is expected.
General Guidelines and Eligibility:

- Both U.S.-based and non-U.S. based (i.e. international) companies engaged in research and development are welcome to apply.
- Awards will be made for either discovery or preclinical and initial clinical development activities (all therapeutic areas relevant to CF).
- To be funded, organizations are expected to provide internal funds that at least match the level of CF Foundation support.
- CFF support may generally not be used for API manufacture or other chemistry, manufacturing and controls activities, clinical trials involving healthy human subjects, support of senior company personnel engaged in administrative roles or Phase III multi-center clinical trials. Costs of these activities may be used as matching funds.
- We recommend projects be conducted in consultation with a CF-identified investigator representing a CFF- accredited Center, CF-supported Therapeutic Development Center, other funded mechanism by the CFF, or other funding agencies, as appropriate for a specific project.
- Funding for Component I will be up to US$300,000 per year for up to two (2) years of support. Funding for Component II will typically be in the $3-5M range for two (2) or more years. At a minimum, awardees are required to provide matching funds equal to the amount requested in the application. Matching funds by the awardee cannot be “in-kind” but must be actual costs incurred by activities of this project.
- Funding is subject to CFF’s availability of funds and the terms of the award.

Approved awards will be subject to monitoring by a Project Advisory Group (PAG), whose membership is approved by CFF. If applicable, the PAG will determine overall performance of the project. If applicable, the PAG will report, in writing, to CFF on a periodic basis usually on a quarterly basis. Milestone completion, and subsequent funding is dependent on the PAG’s review and approval.

- **Milestones**: Applications should contain milestones that are objective achievements demonstrating forward progress for the therapeutic approach and the appropriate timetable for completion of each.

Continued funding for the project will be, in part, based upon milestone attainment.

- **Payback**: If a TDA leads to the marketing of a new intervention, CFF will receive reimbursement for its support. Terms will be negotiated prior to finalizing the award, and will typically include reimbursement, plus a multiple following successful regulatory marketing approval, and/or a percentage of net sales. CFF may also require certain rights to take the product forward in the event that the awardee elects not to advance the product.
III. REVIEW AND AWARD

Applications will be evaluated based on the following:

- The soundness and technical merit of the proposed approach
- The qualifications of the Principal Investigator (PI), supporting staff and CF collaborators
- The relative importance of the proposed intervention to CF care
- The potential of the proposed research for commercial application
- The appropriateness of the budget requested
- The adequacy and suitability of the facilities and research environment
- The adequacy of milestones to assess overall performance of the project

CFF will notify applicants once a funding decision has been made [typically within four (4) months after receiving a full funding application]. Applications and Letters of Intent are accepted throughout the calendar year, although it is highly unlikely that submissions not received by October 31 will be funded in that calendar year.

All successful awardees will be required to execute an agreement specifying the Terms & Conditions of an award before funds are made available.

IV. LETTER OF INTENT (LOI) SUBMISSION GUIDELINES

CFF requires that investigators and/or companies who seek support from the Foundation for TDA applications submit a LOI in advance of a full funding application.

The LOI must be submitted at proposalCENTRAL: https://proposalcentral.altum.com/
The LOI will be considered incomplete if it fails to comply with instructions, or if the submitted material is insufficient to permit adequate review. CFF reviews LOIs electronically, and only the documents submitted online at proposalCENTRAL will be reviewed. All required templates are available for download at proposalCENTRAL.

Following CFF review of the LOI, applicants will receive a notification email either via proposalCENTRAL or from a CFF member of the TDA Committee indicating whether the LOI was approved or declined.

First-time applicants must register to create a user name and password for proposalCENTRAL and will need to complete a profile before applying. If you are already registered and cannot remember your password, click on the “Forgot Your Username/Password?” link below the “Application Login” fields.

Note: Use the Customer Service link on the top right of each screen as needed.

Once logged in, the award opportunities will be listed on the opening screen.