Path to a Cure

Therapeutics Development Awards

Letter of Intent (LOI) and Full Application

POLICIES AND GUIDELINES

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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.

The Path to a Cure is the Foundation’s ambitious research program to support development of treatments for the underlying cause of the disease and, ultimately, a cure for every person with CF. The Foundation is making an initial commitment of $500 million through 2025 for these efforts and is challenging potential collaborators to submit proposals that will accelerate the pace of progress for CF drug discovery and development.

II. PATH TO A CURE OVERVIEW

Although significant breakthroughs are helping people with CF live longer and healthier lives, there are still many individuals who will not benefit from CFTR modulators. Therefore, the Foundation is prioritizing efforts to identify innovative therapeutic approaches for these people with CF.

Industry programs supported through the Path to a Cure will focus on two core strategies to address the underlying cause of CF:

- Restoring CFTR protein when none exists, including that from nonsense mutations
- Fixing or replacing the underlying genetic mutation to address the root cause of CF through gene replacement/transfer, gene editing approaches, or stem cell therapy.

Priority areas include but are not necessarily limited to the following:

PRECLINICAL

- Identify viable readthrough compounds and other strategies to support development of treatments for people with nonsense mutations.
- Platform development for gene delivery and editing strategies

TARGETS

- Develop methods to target appropriate cell(s) in the lung, pancreas and intestine that can be used for CF genetic-based therapies.
DELIVERY

- Develop vectors that efficiently target and deliver genetic cargo to the correct cells in the correct tissue without inducing an immune response (allowing for re-administration).

EDITING

- Develop effective gene editing methods to correct mutations in the CFTR gene that are amenable to clinical development.

CLINICAL STUDIES

- Advance treatments to the clinic with a high potential to benefit people with CFTR nonsense and rare mutations that are not correctable by CFTR modulators.

Any commercial programs developing therapies or therapeutic platforms with the potential to advance these goals will be seriously considered.

Funding Approach:

The level of awards given through the Path to a Cure Initiative are expected to be substantially higher than those through the Component I and II of other CFF Industry TDAs; up to tens of millions of dollars. The structure of CF Foundation investment in the program is flexible and may include milestone-based payouts similar to the TDA program, equity investment, or other strategies beneficial to both parties. Contract terms are negotiated prior to finalization of the agreement.

In addition to funding, the Foundation offers awardees a range of resources to de-risk CF drug discovery and development. This includes access to a robust community of leading academic researchers, preclinical CF-related model systems, infrastructure for preclinical development, and access to the world’s largest network of CF clinical trial sites. Additionally, consulting advice on trial design is supported through the Therapeutics Development Network Coordinating Center.

Investigators and/or companies who seek support from the Path to a Cure should contact the CF Foundation to discuss the research program and its applicability to the program. This may be followed by submission of a Letter of Intent (LOI) in advance of a full funding application.
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 may be made for either discovery, platform development for gene delivery and editing strategies, preclinical and/or clinical development activities.
- It is recommended that projects be conducted in consultation with CF scientists/investigators knowledgeable in the specific aspects of the project.
- 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 determined at the time of contract negotiation. Milestone completion and subsequent funding, if applicable is dependent on the PAG’s review and approval.
- **Royalties to CFF:** If a research award leads to the marketing of a new intervention, CFF will receive reimbursement for its support, the terms of which will be negotiated prior to finalizing the award, and are generally dependent on the stage(s) of development that is(are) funded, and the magnitude of the award.

III. REVIEW AND AWARD

Applications will be evaluated based on the following:

- The soundness and technical merit of the proposed approach
- The expertise of company personnel, qualifications of investigators involved with the project, 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

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.

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 research proposals to submit a LOI in advance of a full funding application. The LOI process may be bypassed with prior CFF approval.
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.