Cystic Fibrosis Foundation

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Mission

The Cystic Fibrosis Foundation (CFF) is a nonprofit donor-supported organization dedicated to the development of new drugs to fight cystic fibrosis (CF), improving the quality of life for those with CF, and ultimately to finding a cure. The foundation funds more CF research than any other organization and has been the driving force behind most of the cystic fibrosis drugs available today. Founded in 1955, it is one of the oldest and most established nonprofit funders of disease research and has served as a model for other medical venture philanthropies. By aggressively investing in promising drug development research with biotech companies and scientists from around the world and pursuing several research strategies at once, CFF has built a pipeline of approximately 30 potential new CF therapies. Part of the foundation's "formula for success" is its ability to attract scientists from new fields to join in the fight against cystic fibrosis.

Strategy/Approach

Finding new medicines to fight, and ultimately cure, CF is the driving force behind all of the foundation's activities. Key programs include:

- **Research Development Program (RDP)** – Established to attract top-notch scientists to CF research, the RDP is a network of 11 research centers of excellence throughout the United States. Since its inception in 1980, the RDP has helped move CF research forward more quickly by encouraging cross-institutional, cross-disciplinary collaboration. RDP centers pool the intellectual resources of scientists and serve as core supply centers, sharing tools with researchers around the world. Scientists at RDP centers also have the opportunity to communicate directly with medical teams at **CFF-accredited care centers** to better understand how their research at the bench translates into therapies.
- **Therapeutics Development Network (TDN)** – A nationwide network of nearly 80 cystic fibrosis **clinical research centers** that specialize in conducting clinical trials to evaluate the safety/efficacy of new CF therapies. Established in 1998 as a subset of the foundation's Care Center Network, TDN centers work together to promote quality, safety, and efficiency in CF clinical trials by centralizing and standardizing the research process. It is composed of foundation-accredited care centers, a central coordinating center at Seattle Children's Research Institute, and laboratories that specialize in interpreting CF outcome measures.
- **Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT)** – Established in 2000, CFFT is the nonprofit drug discovery and development affiliate of the foundation. CFFT supports and governs activities related to CF drug discovery through the many stages of drug development and clinical evaluation.

Research Portfolio

The foundation's research is organized into three broad areas: 1) investigator-initiated research, 2) multidisciplinary research centers, and 3) clinical research centers. A pioneer of the multidisciplinary approach to research – which has since been adopted by other disease organizations – the foundation fosters collaboration across fields (molecular biology, immunology, medicinal chemistry, etc.) to find new strategies to tackle the disease. It also supports efforts by researchers to translate knowledge from basic science laboratories into potential therapies and offers numerous training opportunities. Grant opportunities include:

- **Pilot and Feasibility Awards** – For developing and testing new hypotheses/methods and to support promising new investigators as they establish themselves in CF-relevant research areas.
  - *Up to $40,000 per year (plus 8 percent in indirect costs) for two years*
- **Research Grants** – Intended to encourage the development of new information that contributes to the understanding of the basic etiology and pathogenesis of CF. Information derived from these studies often leads to submission to other funding agencies, such as the National Institutes of Health.
  - *Up to $90,000 per year (plus 8 percent in indirect costs) for two years*
- **Clinical Research Awards** – CFFT offers competitive awards to support clinical research projects directly related to CF treatment and care.
  - *Single-center grants – Up to $100,000 per year (plus 8 percent in indirect costs) for a maximum of three years*
  - *Multi-center grants – Up to $225,000 per year (plus 8 percent in indirect costs) for a maximum of three years*
- **LeRoy Matthews Physician/Scientist Award** – Up to six years of support for outstanding, newly trained pediatricians and internists (MDs and MD/PhDs) to

complete subspecialty training, develop into independent investigators, and initiate a research program.
    - *Ranges from $48,000 in year one to $76,000 in year six*
- **Harry Shwachman Clinical Investigator Award** – This three-year award is intended to facilitate the transition from postdoctoral training to a career in academic medicine, with an active focus on CF-related areas.
    - *Up to $76,000/year plus $15,000 for supplies (indirect costs not allowed)*
- **CFF/NIH-Unfunded Award** – Supports excellent CF-related research projects that have been submitted to and approved by the NIH but cannot be supported by available NIH funds.
    - *Ranges from $75,000 to $125,000/year for up to two years*
- **Training Grants**—CFF offers a variety of competitive awards for individuals interested in careers related to cystic fibrosis research and care.
    - *Postdoctoral Research Fellowship, Clinical Fellowship, & Student Traineeships*

Partnership Practices

The foundation engages in corporate, academic, and nonprofit alliances.

CFF partners with biotech and pharmaceutical companies. Through CFFT, the foundation provides companies and academia with investment capital during the early phases of **drug research through several stages of evaluation.** A few examples include:

- **Kalydeco™** – This CFTR Modulation therapy, which treats the underlying genetic cause of CF in a small subgroup of CF patients with a specific mutation, was approved by the FDA in January 2012. The drug was developed by Vertex Pharmaceuticals, Inc. with significant scientific, clinical, and financial support from the foundation.
- **Compacted DNA (PLASmin™)** – This gene therapy, currently in Phase I trials, is sponsored by Copernicus Therapeutics, Inc. and partially funded by CFFT.
- **Ciprofloxacin DPI** – The U.S. multi-center Phase II trial for this anti-infective was sponsored by Bayer and conducted in the Therapeutics Development Network of CFFT.

Beyond these examples, the foundation has engaged many other corporate partners in CF therapy development, including PTC Therapeutics, Pharmaxis, Gilead Sciences, Genentech, Kalobios Pharmaceuticals, APT Pharmaceuticals, GlaxoSmithKline, and Rempex Pharmaceuticals.

Financials

The Cystic Fibrosis Foundation is a 501(c)(3) nonprofit health organization. The most recent financial information available is from 2012.[1]

Year ending 12/31/12:

- Revenue: $297,680,022
- Assets: $350,629,883
- Grants: $73,053,292 ($1,212,242 for grants to individuals)
- Expenditures: $125,721,614

Key Accomplishments

- In 1989, CFF-supported scientists discovered the defective gene that causes cystic fibrosis – an important breakthrough on the road to a cure.
- The foundation played an integral role in the development and FDA approval of four therapies that are now a routine part of treatment regimens for many with CF.
- The foundation is actively supporting nearly 30 potential new treatments currently in development, more than in the entire history of the disease.
- Cystic Fibrosis Foundation Therapeutics (CFFT), a nonprofit research affiliate of the foundation, was established in 2000 to govern drug discovery and development efforts.
- In 2000, foundation-supported scientists mapped the entire genetic structure of the most common cause of CF lung infections — the *Pseudomonas aeruginosa* bacterium. Researchers can identify the function of specific genes and find ways to turn off the bad ones.
- In 2012, the FDA approved Vertex Pharmaceuticals' Kalydeco to treat a small subgroup of cystic fibrosis patients with a specific genetic mutation. The drug was developed in collaboration with CFF, which provided funding and information about the cystic fibrosis patient population and helped recruit patients for clinical trials.
    - *"The unique and mutually beneficial partnership that led to the approval of this new therapy for some CF patients serves as a great model for future drug development and patient group collaboration moving forward," said Dr. Janet Woodcock, director of the FDA's Center for Drug Evaluation and Research*
- Kalydeco is currently in a stage II clinical trial testing to see if it could be used in combination with VX-661 to treat a broader population of CF patients.

Leadership

The foundation is governed by a Board of Trustees whose volunteer members serve independently and without compensation. It has more than 500 employees in 80 chapters

[1] Information obtained from 2012 Annual Report http://www.cff.org/UploadedFiles/aboutCFFoundation/AnnualReport/2011-Annual-Report.pdf, October 2012

and branch offices across the country and is managed by corporate officers and staff based in Bethesda, Md.

- **President and CEO**: Robert J. Beall, PhD, rjb@cff.org
- **Executive VP and COO**: C. Richard Mattingly
- **Executive VP for Medical Affairs**: Preston Campbell, III, MD
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