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**STATEMENT OF ROBERT J. BEALL, Ph.D**

**PRESIDENT AND CEO**

**CYSTIC FIBROSIS FOUNDATION**

**BEFORE THE HOUSE COMMITTEE ON SMALL BUSINESS**

**ON THE SMALL BUSINESS INNOVATION RESEARCH PROGRAM**

**JANUARY 29, 2008**

Thank you, Chairwoman Velazquez and members of the Committee, for the opportunity to testify today. I am Robert J. Beall, President and CEO of the Cystic Fibrosis Foundation.

More than twenty-five years ago, when I served at the National Institutes of Health, I had the opportunity to award Small Business Innovation Research (SBIR) grants to pioneering companies. Today, at the CF Foundation, I continue to work with many small businesses who benefit from this successful program. I am pleased to appear before the Committee today on a topic of central importance to me and the Foundation.

We at the CF Foundation are dedicated to utilizing innovative strategies for the development of new therapies for cystic fibrosis (CF), including encouraging partnerships among diverse research and development entities in the public and private sectors. We consider small business entities key players in the fight against cystic fibrosis, and their participation in our efforts has been facilitated by the Small Business Innovation Research (SBIR) program. On behalf of the CF Foundation, I appreciate the opportunity to comment on how the SBIR program can be more effective for the development of new therapies for CF and other serious and life-threatening diseases, including those that are also considered orphan diseases.

### **The Research and Development Mission of the CF Foundation**

The CF Foundation has a multi-faceted research program that strategically invests in basic research and in companies that are engaged in the development of new CF therapies. Through this aggressive approach, we have contributed to a significant improvement in the survival of those with CF. Because of research supported by the CF Foundation, the median age of survival for people with CF has increased dramatically from less than six years in 1955 to 37 years today.

This achievement is obviously not adequate for those with CF, who face a disease that requires rigorous daily treatments and that has a profound impact on quality of life.

The CF Foundation is supported by the community of individuals with CF and their families and friends and by many other committed individuals and organizations who contribute generously to the mission to find a cure.

We have developed an innovative research model that is described as venture philanthropy. This means that the Foundation directly invests, much as a venture capitalist would, in research and development of new CF therapies. We have invested over \$660 million in our research and medical programs, and in 2008, we will invest \$27 million in CF research at biotechnology companies for the development of new drugs.

Our program is multi-faceted and comprehensive because we collaborate with many different partners in the public and private sectors. It is also complex because we are focusing on a wide range of research issues and potential products to address the many ways in which CF affects patients. Our research and development pipeline includes efforts related to gene therapy, efforts to modify the defective gene and its protein that cause CF, ion transport restoration, mucus regulation, anti-inflammatory therapy, anti-infectives, transplantation, and nutrition.

We are fortunate to have so many therapeutic targets to pursue, yet we are racing the clock to develop new CF therapies. Despite our successful fundraising efforts, we cannot pursue all of the promising research opportunities before us without help and without partners.

The SBIR program reflects our fundamental philosophy of creating viable and creative partnerships to accelerate the development of new therapies. SBIR grants are particularly important for companies pursuing the early discovery phase of drug development – the most difficult time to secure funding.

SBIR grants serve as an incubator for innovative early-stage research, much like our own venture philanthropy model. Like our model, SBIR grants provide the critical support companies need to prove their research concept. Once a company passes this hurdle, investors are more willing to invest to bring therapies to market. For people with cystic fibrosis, this model continually adds new drug candidates to the development pipeline, increasing the chances of producing effective therapies and finding a cure for this disease. It also shortens the time it takes to bring a new drug to market. In the past 14 years, four cystic fibrosis therapies have been made available with the support of the CF Foundation to treat this disease: Pulmozyme®, TOBI®, azithromycin, and hypertonic saline. The time taken for the Foundation to help develop Pulmozyme® – five years from test tube to cystic fibrosis patients – was less than half the industry average.

We urge that the SBIR program be reauthorized, with minor but important modifications, so that it can continue to foster the involvement of small businesses in research and development.

### **Create a Set-Aside for Rare Disease Research**

Twenty-five years ago, Congress recognized the need to encourage research and development related to rare diseases – those affecting fewer than 200,000 Americans – by passing the Orphan Drug Act. In the years since the law was enacted, Congress has reaffirmed its commitment to research on rare diseases. Although the existing federal programs have had a positive impact on private sector involvement in rare disease research and development, there is still significant reluctance to be involved in orphan disease research.

There are substantial risks related to research and development of any new therapeutic product. For orphan diseases, the obstacles that apply to developing all new therapies are even higher, and the rewards for development of an orphan drug are limited by the size of the market. This combination of factors argues against industry involvement in orphan drug research, so incentives must be created for these companies.

The venture philanthropy efforts of the CF Foundation have been essential in attracting companies to orphan drug research or keeping those companies in the field. Our support alone, however, is not adequate to retain the attention of those who have shown interest in CF research, and to encourage new entities to join the effort. I also want to emphasize that there are many diseases that do not enjoy the strong private sector support and the venture philanthropy support that the CF Foundation provides for cystic fibrosis.

The CF Foundation urges the Committee to set aside a portion of SBIR funds at the National Institutes of Health (NIH) for support of biotechnology companies that are focused on orphan disease research and development. We would recommend a set-aside of 10 percent of SBIR funds at NIH. This approach is fully consistent with the

fundamental goals of the SBIR program to increase the commercial application of federally supported research and to stimulate technological innovation in the private sector. We also believe this modest targeting of funds to rare diseases might have the added benefit of encouraging applications from small business entities that have a specific interest in rare diseases but have never been SBIR applicants.

### **Address Ownership Limits**

We note with great concern that in recent years there has been a decline in the applications to NIH for SBIR grants. We understand that this decline is generally attributed to the impact of an eligibility standard that requires companies to be at least 51 percent owned and controlled by one or more individuals who are citizens of, or permanent resident aliens in, the United States. The standard has resulted in the disqualification of small business entities that have succeeded in attracting venture capital investment and, therefore, cannot meet the individual ownership rule.

Although the Foundation supports efforts to prevent abuses of the SBIR program, we fear that the individual ownership rule is having an unintended and regrettable impact by disqualifying small business entities that could play an important role in research and development of new therapies for CF and other diseases.

The current ownership rule, in effect, winnows out many of the successful, proven companies that have demonstrated the ability to develop innovative research into therapies for patients. It would seem this is the type of success that a governmental program should reward and invest in, rather than disqualify. Much as our own venture philanthropy model opens a successful company's eyes to the possibility and promise of cystic fibrosis therapeutic development as a valuable asset for their business and our patients, a SBIR grant can enable a company – with the right capabilities and a proven track record – to pursue a project they might otherwise not consider because of its initial risk.

PTC Therapeutics is one of our great partners in the effort to develop treatments for cystic fibrosis. The company has multiple promising CF therapies in development, including PTC-124, an innovative oral drug to treat the basic genetic defect of cystic fibrosis, and potentially 2400 other genetic disorders. The company, like several of our partners, received an SBIR grant for the early discovery phase of the drug that ultimately became PTC-124. The development of this ground-breaking therapy depended on the SBIR grant as it was considered too risky to be funding by private venture capital funding.

As PTC-124 moved through development, the company applied for and received a larger SBIR grant to continue the work. Unfortunately, they could not accept the funding because of the current ownership rules and had to find other funding sources, including the Cystic Fibrosis Foundation as well as others. In this case, the current ownership rule slowed this innovate research and placed this promising drug at risk of derailment.

As a Foundation dedicated to investing in the best opportunities for research at innovative companies, we consider the interest of other venture capitalists in a company as a signal of ability and a vote of confidence. It is a marker that the company may have the personnel, skills, and competence to function as a good research and development partner. We think the SBIR program would do well to rethink the ownership rules to ensure that it can attract the widest possible range of competent SBIR applicants.

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The Cystic Fibrosis Foundation lends its strong support to reauthorization of the SBIR program. This program facilitates partnerships that are critical for development of new treatments for CF and hundreds of other diseases. In an age of limited federal resources, we applaud the SBIR program because it facilitates collaboration between public and private sectors in an efficient manner.

We would recommend two minor modifications of the program: 1) a modest targeting of NIH SBIR funds to rare diseases research and development, and 2) changes in the individual ownership rules so that successful research and development companies will not be disqualified from SBIR eligibility because they have attracted venture capital funding.