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**STATEMENT OF MARY B. DWIGHT**

**VICE PRESIDENT OF GOVERNMENT AFFAIRS**

**CYSTIC FIBROSIS FOUNDATION**

**BEFORE THE HOUSE COMMITTEE ON SMALL BUSINESS**

**ON THE SMALL BUSINESS INNOVATION RESEARCH (SBIR) PROGRAM**

**JUNE 17, 2009**

Statement of Mary B. Dwight  
Vice President of Government Affairs  
Cystic Fibrosis Foundation  
Before the House Committee on Small Business  
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Chairwoman Velazquez, Congressman Graves and members of the Committee, thank you for the invitation to testify on behalf of the Cystic Fibrosis Foundation. It is my privilege, in particular, to speak about the important role of the Small Business Innovation Research (SBIR) program in the development of therapies for cystic fibrosis (CF) and other serious and life-threatening illnesses.

The CF Foundation is *the* leader in the search for a cure for cystic fibrosis. To develop new therapies for CF and accelerate progress toward a cure, it has pioneered a strategy of “venture philanthropy.”

We made the decision to pursue a venture philanthropy model out of necessity. First, we could not accept the fact that children born with CF in 1955 often did not live to attend elementary school, and we recognized that for people with this disease, every second counts.

Second, while our progress since the Foundation’s founding has been steady, it is not adequate. The life expectancy for people with CF is still far below the national average, and children are still dying each day from this disease.

Third, because the CF population is small, those companies that are successful in product development are far less likely to pursue the development of CF drugs that have an exceedingly small market for such products.

The needs of CF patients and the economics of CF drug development necessitated an aggressive and creative approach. By applying business concepts and collaborating with the biotechnology and pharmaceutical industry, the CF Foundation has quickened the pace of drug discovery and development. In the past 14 years, four cystic fibrosis therapies have been developed with support of the CF Foundation: Pulmozyme<sup>®</sup>, TOBI<sup>®</sup>, azithromycin, and hypertonic saline. The time taken for the development of Pulmozyme<sup>®</sup> – five years from test tube to use by cystic fibrosis patients – was less than half the industry average. In addition, our research and development program has yielded a robust pipeline of 30 potential therapies.

At the heart of this effort is the Therapeutics Development Program, through which the CF Foundation functions much as a venture capitalist would, directly investing in the research and development of new CF therapies. We have invested over \$660 million in our research and medical programs, and in 2009 we will invest \$26 million in CF research at biotechnology companies for the development of new drugs. Our venture philanthropy approach, recognized by the National Institutes of Health (NIH) and by

publications such as *Business Week*, *Harvard Business Review*, *USA Today*, *Forbes* and *The New Yorker*, encourages private firms to become engaged in CF research and development by addressing some of the risk associated with the small patient population and the resulting limited market.

Through this aggressive research program, the CF Foundation has made significant progress in the treatment of CF. The median age of survival is now more than 37 years, more than double what it was just 25 years ago, and more than seven times what it was when the CF Foundation was established in 1955. Yet we have more to do. The pipeline of 30 potential therapies includes some aimed at treating the symptoms of the disease and others intended to correct the genetic defect that causes CF. Testing these therapies and others will require substantial resources, and it is imperative that all potential sources of funds be available to advance research and development of these treatments.

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. Other research foundations engaged in basic, translational and clinical research are facing challenges similar to ours. Those foundations that are successfully moving new compounds through testing are struggling during the recession, and those with fewer compounds in clinical tests face challenges in financing their basic research efforts intended to identify such compounds. In short, all research foundations are attempting to meet the needs of their patients for new therapies in a daunting economic environment. The need for strong partners, including partners who have SBIR funding, has never been greater.

The SBIR program reflects our fundamental philosophy of building 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, a period of research for which it is especially difficult to secure funding. SBIR-funded research entities often enjoy a productive synergy between SBIR funding and venture capital funding. Small biotechnology companies are able to use SBIR funds in combination with funding from other sources, including non-profit organizations like the CF Foundation, to overcome the most vulnerable stages of development. Likewise, biotechnology companies that secure SBIR funds make themselves more attractive to outside investors.

SBIR grants often offer critical support for innovative early-stage research, much as our venture philanthropy support does. Like our model, SBIR grants provide the 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.

The SBIR program has been a success, and the need for it has never been greater. We urge some changes to the administration of the program to ensure that it meets its fundamental goals and supports research in fields that would otherwise be neglected because of small markets or obstacles to research.

The current ownership rules for small businesses must be revised. In addition, the program rules should be amended to dedicate a percentage of SBIR funds to research on orphan diseases, as early stage research on these diseases is understandably not an attractive target for pharmaceutical and biotechnology companies. We applaud the House Small Business Committee for its past and ongoing efforts to address each of these issues.

### **Amend Ownership Rules**

One of the chief obstacles in our quest for a cure for CF is the availability of adequate financing for research and development of new treatments. Historically, access to SBIR funding has contributed to the ability of biotechnology companies to remain engaged in CF product development.

Unfortunately, in recent years the CF Foundation has directly observed a decline in the number of firms that are engaged in CF research and are also receiving SBIR funding. Some firms with promising CF therapies report that they are disqualified from receiving SBIR funds due to their receipt of venture capital support. Although there appears to be some disagreement about the overall impact of the ownership rule on the number of SBIR applications submitted by medical research entities, the direct experience of the CF Foundation suggests that the impact has been significant.

While we understand the need to ensure that the SBIR program truly supports small business entities and protects against abuses of the program, we find that the ownership rule has had the no doubt unintended impact of disqualifying from the SBIR program those small business entities that hold the greatest promise of partnering with the CF Foundation to bring new therapies to market. It has been our experience that those small businesses that are able to obtain venture capital funding are our strongest partners, yet those partners still need both CF Foundation and SBIR support to be able to advance their development work. As we previously described, our therapeutic development model requires support from a variety of sources. The loss of any source of financing can have a significant adverse impact.

We applaud the efforts of the Committee to address the ownership rule through legislative change and agree that the focus should be on the control of the small business rather than strictly on venture capital investment. We can again cite the experience of the CF Foundation to underscore that our potential partners that have lost SBIR eligibility clearly meet other requirements of a small business entity, including the fact that they retain independence in management and other decisions. In our experience, the SBIR ownership rule is disqualifying small businesses that are the intended target of the program.

Our experience is supported by findings from a National Research Council report on venture funding and the NIH SBIR program. This report concluded that the ownership

rule disproportionately affects firms with demonstrated potential for commercializing their drug products. The report found that firms that had venture funding were less likely to bring their products to market than those firms that were not venture-funded. When the venture-funded firms did commercialize their products, they were much more likely to generate substantial sales from their SBIR-funded projects.

The CF Foundation considers its best partners those that have been able to attract venture capital investment and SBIR funding. Venture capital funding, like SBIR funding, is often an indication that a small company has a strong and sophisticated management and development team. This factor, combined with the availability of venture capital and SBIR funds to supplement those provided by the CF Foundation can define these firms as solid R&D partners, with an increased likelihood of success.

### **Provide a Special Focus for Orphan Diseases**

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. Since that time, Congress has reaffirmed its commitment to this research by creating incentives to companies that develop orphan drug products and providing discretionary funding for research on orphan diseases. These initiatives have proven successful, as measured by the development of new therapies for a number of rare diseases.

The existing rare disease research programs could be significantly strengthened by ensuring the availability of funds for orphan disease research and development through SBIR. This funding would help reduce a financing problem facing companies that are moving beyond basic research on an orphan drug product but are not yet able to commercialize their product.

The risks related to the research and development of new therapeutic products are substantial and far greater for development of an orphan drug than a conventional drug. The rewards for development of an orphan drug are also 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 drug development companies.

The venture philanthropy efforts of the CF Foundation have been essential in attracting companies to orphan drug research as well as keeping companies in the field. Our support alone is not adequate to retain the attention of those who have shown interest in CF research or to encourage new entities to join the effort. Although we pride ourselves on our successes, we are acutely aware that there are many diseases that do not enjoy the strong private and venture philanthropy support that the CF Foundation provides for cystic fibrosis.

An appropriate solution to this problem would be to set aside a portion of SBIR funds at NIH for support of biotechnology companies focused on orphan disease research and

development. We believe that a set-aside total of 10 percent of SBIR funds at NIH would represent an important investment in rare disease research. 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.

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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. Targeting a modest portion of SBIR funds to rare disease research holds the potential of attracting new companies to rare disease research and providing new therapies to patients with few options and in dire need of new treatments.

We look forward to the opportunity to talk to you in greater detail about these proposals.