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Organization: Cystic Fibrosis Foundation
Written Testimony for Fiscal Year 2012 Appropriations

Subcommittee: Agriculture, Rural Development, Food and Drug Administration, and
Related Agencies

Agency: Food and Drug Administration

On behalf of the Cystic Fibrosis Foundation and the approximately 30,000 people with cystic fibrosis (CF), we are pleased to submit the following testimony regarding the fiscal year 2012 appropriations for the Food and Drug Administration (FDA)'s review of rare disease treatments.

ABOUT CYSTIC FIBROSIS

Cystic fibrosis is a life-threatening genetic disease for which there is no cure. People with CF have two copies of a defective gene, known as CFTR, which causes the body to produce abnormally thick, sticky mucus that clogs the lungs and results in fatal lung infections. The thick mucus in those with CF also obstructs the pancreas, making it difficult for patients to absorb nutrients from food.

Since its founding, the Cystic Fibrosis Foundation has maintained its focus on promoting research and improving treatments for CF. More than thirty drugs are now in development to treat CF; some treat the basic defect of the disease, while others target its symptoms. Through the research leadership of the Cystic Fibrosis Foundation, people with CF are living into their 30s, 40s and beyond. This improvement in the life expectancy for those with CF can be attributed to research advances and to the teams of CF caregivers who offer specialized care. Although life expectancy has improved dramatically, we continue to lose young lives to this disease.

The promise for people with CF lies in research. In the past six years, the Cystic Fibrosis Foundation has invested over \$1 billion in its medical programs of drug discovery, drug development, research, and care focused on life-sustaining treatments and a cure for CF.

This testimony focuses on the funding the Food and Drug Administration needs to quickly and efficiently review treatments for CF and other rare diseases so they can swiftly move into the hands of the patients who need them.

SUSTAINING FUNDING FOR RARE DISEASE DRUG REVIEW AT THE FDA

Cystic Fibrosis Foundation Drug Development Model

The Cystic Fibrosis Foundation has been recognized for its unique research approach, which encompasses everything from basic research through Phase 4 post-marketing drug safety monitoring, and has created the infrastructure required to accelerate the development of new CF therapies. As a result, we now have a pipeline of more than thirty potential therapies which are being examined to treat people with CF.

One such treatment is VX-770, a drug being developed by Vertex Pharmaceuticals that was discovered in collaboration with the CF Foundation. This promising therapy actually targets the genetic defect that causes CF in patients with a particular mutation of cystic fibrosis, as opposed to only addressing symptoms of the disease. In late February we learned that Phase 3 clinical trial data of VX-770 showed profound improvements in lung function and other health measures in CF patients, and a New Drug Application is expected to be submitted to the FDA for review later this year. This new treatment is a direct result of the Foundation's innovative research agenda, advancing from bench to bedside through the Foundation's research program which speeds the creation of new CF therapies.

Funding for Rare and Orphan Disease Drug Review

In order to encourage the swift review of drugs for CF and other rare diseases, we urge the Committee to recommend sufficient funding for the Food and Drug Administration, particularly the Center for Evaluation and Research (CDER)'s Office of New Drugs. Reducing FDA funding to FY08 levels, as has been proposed, would set rare drug review and approval back at a time when effective treatment for some of our most deadly diseases is sorely needed.

In order to be effective, the FDA needs not only an adequate number of reviewers of new treatments, but also those with the appropriate skills and expertise, particularly for rare diseases like cystic fibrosis. Additional support for the FDA through increased funding not only assures that the nation has a safe and effective supply of drugs and devices, but also that the agency can give the necessary attention to reviewing treatments that treat small populations but serve specific unmet medical needs, such as new CF drugs.

The Subcommittee and Congress should be commended for recent funding increases for the FDA. Nonetheless, the agency continues to face resource constraints. Its workload has increased due to threats from bioterrorism and other public health emergencies. Even with funding increases in recent years, FDA's appropriation supported about 9,100 full-time employees in FY 10. This is the same personnel level as 1994, a time in which FDA faced fewer challenges and its job was considerably less complex.

It is now more critical than ever that Congress significantly increase funding for the Center for Drug Evaluation and Research (CDER) at the FDA and for the agency as a whole in FY2012, so that it can meet its statutory obligations to review drugs for safety and efficacy in a timely manner.

Accelerating the Rare Disease Drug Review Process at the FDA

The Cystic Fibrosis Foundation applauds the FDA, and Associate Director for Rare Diseases Dr. Anne Pariser in particular, for their attention to rare disease drugs and sensitivity to the unique challenges posed by the evaluation of these treatments.

FDA review officials have taken steps to improve their scientific expertise for review of therapies to treat rare diseases, and FDA leaders and review staff have been willing to engage in constructive dialogue to address issues with rare disease review. The agency has consistently taken part in productive conversations with medical experts, researchers, clinicians, and patients at the Cystic Fibrosis Foundation, including many of the foremost experts in the world on cystic fibrosis. This collaboration has augmented the FDA’s work, allowing experts in CF to provide the FDA with the information it needs to effectively evaluate new treatments and accelerate the approval process, such as the CF Foundation’s ongoing research into the development of improved tools for Patient Reported Outcomes and measurements of lung function.

However, in many cases the opportunity for public comment is not available if the product in question is not the subject of an advisory committee. In all cases, this public comment period occurs very late in the review process. We recommend that the agency consider establishing a procedure to receive comment from patients and their physicians earlier in the process, at the time of the submission of the Investigational New Drug (IND) application. Receiving such input earlier might be especially useful in defining and addressing the matter of unmet medical need. Because orphan diseases are by definition of limited prevalence, it is generally unlikely that specific expertise in the disease will be available among FDA staff. For that reason, the agency should be willing to inform its review process through early input from experts – both patients and professionals – regarding living with the disease, treating the disease, and developing therapies for it.

Additionally, the Foundation commends the establishment of the new regulatory science initiative, formed by the NIH and the FDA, with the goal of accelerating the development and use of new approaches to evaluate drug safety, efficacy and quality, and urges the Subcommittee to strongly support this type of collaboration. Support for these types of collaborations throughout the national health agencies, including programs like the Therapeutics for Rare and Neglected Diseases Program and the Cures Acceleration Network, leverages the federal investment in new research, facilitating swifter development and delivery of new medical treatments.

The Cystic Fibrosis Foundation’s unique and successful drug development model for creating treatments for a rare disease has helped create a pipeline with more than thirty promising drugs to fight cystic fibrosis, and the Food and Drug Administration has played a critical role in this

process, working with the Foundation as they review treatments and move them into the hands of those who need them. Encouraged by our successes, we believe the experience of the CF Foundation in clinical research can serve as a model of drug discovery and development for research on other orphan diseases and we stand ready to work with the FDA and Congressional leaders. On behalf of the Cystic Fibrosis Foundation, we thank the Committee for its consideration.