CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

December 5, 2014

Margaret Hamburg, M.D.
Commissioner
Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993-0002

Re: Request for Comments on Disease Areas for Patient-Focused Drug Development Meetings

Dear Dr. Hamburg:

The Cystic Fibrosis Foundation is dedicated to finding a cure for cystic fibrosis (CF), a rare genetic disease that affects 30,000 Americans. We pursue our mission by spearheading an aggressive research and therapeutic development program, a model clinical care program, and providing other services to the individuals affected by CF and their families. We have pioneered the venture philanthropy model for investing in CF therapeutic research and development, a model that has produced disease-altering treatments and is fueling additional research to cure CF.

We are writing in response to the request for recommendations for disease areas for patient-focused drug development meetings in 2016-2017. We propose that cystic fibrosis be included on this list.

Cystic fibrosis is a serious chronic disease that affects the activities of daily living. Over the last 30 years, a number of therapies have been developed to address the symptoms of CF. The impact of those therapies has been impressive, as individuals with CF now have a median life expectancy in the early 40's. This is a dramatic improvement from the 1950s, when children diagnosed with CF were not expected to live to attend school. In the last two years, new treatments that address the underlying cause of CF have been approved. Those drugs are available at this time for a small portion of the 30,000 Americans living with CF.

Even with the significant improvements in CF care, individuals with CF must still undertake a daily regimen of care that includes airway clearance, inhaled medicines to help fight lung infections, and pancreatic enzyme supplementation to aid digestion. These treatment steps are still necessary for those who also benefit from the recently approved disease-modifying drugs. In other words, those with CF never take a vacation from their disease. A meeting focused on CF would permit consideration of the daily burden of the disease as well as the unmet medical needs of CF patients.

Improvements in the median life expectancy for those with CF may suggest to some that the treatments for CF are adequate. In fact, this is not true and there remain serious unmet medical needs for those with the disease.

CF affects the lungs, pancreas, and other organs. Individuals with CF progressively lose lung function each year, suffer from digestive issues that can rise to the level of malnutrition, experience serious lung infections, and can develop diabetes and other co-morbid conditions in adulthood or earlier. Trials of disease-modifying therapies that are designed to evaluate the impact of the drug on a single endpoint may not produce data regarding the effects on other symptoms of the diseases. Patients have in recent trials reported significant positive impact on overall quality of life, but that improvement might not be appropriately measured for submission as part of the new drug application. A CF patient-focused drug development meeting would provide an important opportunity for consideration of quality of life issues in CF and their measurement in clinical trials.

The CF community demonstrates each day its commitment to the clinical research enterprise. Individuals with CF willingly and even enthusiastically participate in clinical trials, and some with CF have participated in multiple trials over their lifetime. CF patients as a result can offer significant advice about clinical trials – their structure, enrollment strategies, endpoints, quality of life measurement, and other issues. This advice would be valuable not only for the design and execution of CF trials but would also have implications for trials in other rare disease areas.

CF is not a neglected disease in terms of the research and therapeutic development investment, but there are still lessons to be learned and information to be shared about patient-focused CF research and development. A meeting that focused on CF would yield insights not only about CF research and development but also valuable takeaways for other rare diseases.

We appreciate the opportunity to comment on the patient-focused drug development meeting list. We look forward to publication of the final 2016-2017 list and hope that cystic fibrosis will be included.

Sincerely,

Robert J. Beall, Ph.D.
President and Chief Executive Officer