CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

December 4, 2014

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

Re: Request for Comments on FDA Activities for Patient Participation in Medical Product Discussions

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 through an aggressive research and therapeutic development program as well as through a model clinical care program and other services for 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.

An efficient, effective and well-informed Food and Drug Administration (FDA) is critical to the CF therapy development program. We have aimed to improve the CF research and development program, and we are gratified by the performance of FDA in reviewing and approving the products of our research. Open communication and consultation with CF patients, clinicians and researchers can further inform and strengthen the FDA review process, and we applaud the willingness of the agency to accept advice about patient participation in FDA activities.

***Patient Participation in Advisory Committees and Earlier in Product Development***

FDA has clearly defined a process for identifying and qualifying patient representatives as special government employees to serve as members of advisory committees as well as procedures for patients to participate in public comment periods during advisory committee sessions. Patient representatives can bring an important perspective to the deliberations of

advisory committees, and patients can also bring to advisory committees important perspectives on unmet medical needs and the risk-benefit calculation for a new therapy.

We agree that it is important for patient representatives serving on advisory committees to disclose any potential conflicts of interest. Individuals speaking at advisory committees should also disclose any relationships with product sponsors and their competitors as well as any other research funders, including the CF Foundation. We have no fundamental objections to the standards for participation in advisory committees. However, attendance at advisory committees is not advised for more than one CF patient. Infection control standards argue against more than a single CF patient being at the same public event. The agency can address the infection control limits by permitting and encouraging participation by phone or web conferencing. We encourage the agency to make such procedures routine for those with CF and others who might be governed by similar infection control procedures or who are otherwise homebound.

Although there are benefits to participation in advisory committee deliberations by patient representative committee members and members of the public, there would be added value in consulting with patients early in the product development and review process in addition to the advisory committee process. For example, CF patients can offer advice about their unmet medical needs, their perspective on balancing the risks and benefits of new therapies, measurement of quality of life, the burden of their disease and current treatments for their disease. CF patients are active and enthusiastic participants in clinical trials, and as a result they are able to offer significant advice about trials.

The advice of CF patients could be useful from the earliest stages of discussion between FDA and sponsors regarding product development and clinical trial design through the entire review and approval process. We urge the agency to embrace the potential of patient participation in consultation with FDA in advance of the advisory committee process. We understand that initiating the conflicts of interest process for patient representatives imposes a responsibility and even a burden for FDA, but in the case of CF patients we know there would be no resistance to adhering to conflicts review.

***Consultation with External Experts on Rare Diseases, Targeted Therapies, and Genetic Targeting of Treatments***

The Food and Drug Administration Safety and Innovation Act (FDASIA) includes a provision authorizing consultation with external experts on rare diseases, targeted therapies, and genetic targeting of treatments (FDASIA Section 903). Congress included this provision in the user fee reauthorization in response to concerns of the CF Foundation and other rare disease organizations regarding the challenges that FDA faces in obtaining and retaining staff expertise and knowledge about rare diseases and the many different genetic mutations that might exist

within a rare disease population. In the face of these challenges associated with retaining staff expertise related to rare diseases, Congress authorized a structure for the agency to obtain such expertise through consultation with outside experts.

The FDASIA provision anticipates that FDA will consult with external experts who possess scientific or medical training and who will be qualified as special government employees for the purposes of such consultation. The agency is authorized to develop a list of external experts who have special expertise on rare diseases and a series of issues related to rare diseases, including disease severity, unmet medical needs, the willingness and ability of individuals to participate in clinical trials, an assessment of the benefits and risks of therapies, the general design of clinical trials for rare diseases, and the demographics and clinical description of patient populations.

We urge the agency to take concrete action to implement the expert consultation program anticipated by FDASIA. Moreover, we urge that the provision of the law be considered to include patient representatives among those who have scientific and medical training that qualifies them for consultation by FDA. The experience of living with a serious chronic disease forces patients to learn about their disease, available treatments, research and development of new therapies, and their own unmet medical needs. As we have discussed above, patients could offer valuable advice to FDA during the product development and review process. Including them as outside experts would ensure that FDA has access to a rich and well-informed group of experts.

We appreciate the opportunity to comment on patient participation in the drug review process. We commend the agency for the steps it has taken to date to consult with patients as part of the advisory committee process. We recommend that FDA take steps to engage patients early in the development and review process, to ensure that the agency has access to expertise necessary for the review of drugs in the age of genetically targeted medicines.

Sincerely,

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