

STATEMENT OF ROBERT BEALL, PHD
SUBMITTED TO SUBCOMMITTEE ON AGRICULTURE
COMMITTEE ON APPROPRIATIONS
UNITED STATES SENATE
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Chairman Kohl and Ranking Member Brownback, it is my pleasure to submit this statement on behalf of the Cystic Fibrosis Foundation. We commend the Subcommittee for convening this hearing to consider Food and Drug Administration (FDA) review of products for rare and neglected diseases and to assess the impact of priority review vouchers for tropical diseases. For all of those affected by rare and neglected diseases, an efficient and effective review system is absolutely critical. Delays in the evaluation of safety and effectiveness of promising new therapies for rare diseases adversely impact those affected by these diseases, and we support efforts by the agency to improve its review record as well as the oversight provided by Congress.

The CF Pipeline

Cystic fibrosis (CF) is a disease that affects only 30,000 Americans and 70,000 individuals worldwide. The effects of this disease are severe, despite significant therapeutic advances, outstanding management of the disease by patients and their physicians, and enhanced adherence to standards of clinical care. There is a pressing need for improved therapies for CF, and as new treatments are developed, efficient review is necessary.

Through aggressive investment in and management of the CF therapeutic development program, the Cystic Fibrosis Foundation is now managing a rich portfolio of potential new treatments with more than 30 drugs in the clinical development pipeline. Included in our research efforts are drugs that may correct the genetic defects that cause CF. The CF Foundation is assuming an expansive role in research, supporting basic research, functioning as a venture philanthropist through investment in biotechnology companies for development of new CF therapies, and coordinating CF care quality improvement through a patient registry that includes most CF patients in the nation.

The venture philanthropy effort has yielded a number of potential CF treatments. Our efforts to date have focused on translating basic research findings into agents for clinical testing, coordinating the clinical trials network for testing CF treatments, and removing barriers to participation in trials by CF patients. As promising treatments will

soon emerge from the development pipeline, our attention is increasingly focused on guaranteeing an efficient FDA review process.

We have identified a number of issues that should be addressed to improve FDA review of CF therapies, and we believe that FDA action on these issues would benefit review of all rare disease treatments. These issues include: 1) identification of and regulatory agreement regarding endpoints for approval of rare disease treatments; 2) making widely and readily known the process for validation of biomarkers to identify subpopulations of CF patients who might benefit from therapies approved for other populations; 3) consistency between FDA and the European Medicines Agency, to eliminate difficulties associated with conducting parallel and duplicative trials in orphan populations; and 4) regulatory guidance regarding methods for evaluating supplemental uses of devices, including nebulizers, without undertaking trials that are prohibitive for cost and other reasons. We also encourage the agency to ensure that it receives appropriate expert advice and guidance on rare diseases as products for those diseases are reviewed.

We are encouraged by initiatives that the agency has undertaken to enhance its scientific expertise for review of rare diseases and more generally by the willingness of FDA leaders and review staff to engage in constructive dialogue to address the problems of rare disease review that we have identified.

The joint regulatory science initiative of FDA and the National Institutes of Health (NIH) signals the firm commitment of the agencies to enhance the scientific expertise of FDA review staff. This effort, still a relatively new one, promises to provide special benefits in strengthening the scientific knowledge and experience for rare disease treatment review. In addition, the agency directed important resources and attention to rare disease treatments by naming a lead reviewer on rare diseases. We have also found the agency to be willing to engage in constructive dialogue to address other problems posed by rare disease review and those issues that are specific to CF product review.

We applaud the Subcommittee for turning its attention to FDA review of treatments for rare diseases and to evaluating initiatives or programs that might enhance such review. The priority review voucher program for rare diseases deserves a fair and full evaluation, to ensure it is meeting program goals and to assess whether its expansion to rare diseases might be appropriate. We support a collaborative and constructive approach to enhancing FDA review and are pleased to see that spirit of cooperation in the efforts of the Subcommittee.

Thank you again for this opportunity to submit this statement.