

NCATS Statement
March 2011

Dear Dr. Collins,

On behalf of the Cystic Fibrosis Foundation, I want to thank you for this opportunity to offer our comments on the National Institutes of Health (NIH)'s proposed National Center for Advancing Translational Sciences (NCATS) and for your untiring efforts to bridge the "Valley of Death" to advance treatments for all diseases, including cystic fibrosis.

The Cystic Fibrosis Foundation applauds the formation of the National Center for Advancing Translational Sciences at NIH. We wholeheartedly support the mission of this new center, which will bring existing translational sciences programs at NIH under one roof and foster new and innovative methods for turning basic science discoveries into potential treatments for some of our most devastating diseases.

Creating a focal point for translational sciences at NIH will ensure a more robust, integrated, and systematic approach to this discipline. The new center will help convene cross sector collaborations between industry, government, and academia to advance drug development. By providing services and resources for high throughput screening, assay development and preclinical modeling, this new center will give industry, academia and others access to the tools they need to jump start the development of treatments that otherwise would prove too risky and cost prohibitive to cultivate.

We hope NIH will use the Cystic Fibrosis Foundation as a resource and a model to accelerate the development of treatments through NCATS. As you know, the CF Foundation has fostered a widely renowned drug development model that performs many of the functions outlined above. This approach 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. The CF Foundation's Therapeutics Development Network (TDN), a key element of CFF's model, has been cited as an exemplar for NIH's Therapeutics for Rare and Neglected Diseases (TRND) program, through which NIH partners with outside entities to move candidate drugs through the pipeline and creates collaborations among researchers in diverse disciplines and areas of expertise.

As a result of the CF Foundation's efforts, we now have a pipeline of more than thirty potential therapies that 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 potential 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.

The Cystic Fibrosis Foundation's successes can serve as a map for the development of new treatments for other diseases, and we urge NIH to use CFF's considerable expertise as a resource. Once again, we commend the National Institutes of Health for its planned formation of the National Center for Advancing Translational Sciences, and thank you for this opportunity to share our thoughts.

Sincerely,

Robert J. Beall, Ph.D.
President and CEO
Cystic Fibrosis Foundation
Bethesda, Maryland