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On behalf of the Cystic Fibrosis Foundation and the 30,000 Americans with cystic fibrosis (CF), we are pleased to submit the following testimony with our requests for Fiscal Year 2012 Labor, Health and Human Services, and Education Appropriations

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 CF Foundation has maintained its focus on promoting research and improving treatments for CF. More than 30 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. A greater investment is necessary, however, to accelerate the pace of discovery and development of CF therapies.

SUSTAINING THE FEDERAL INVESTMENT IN BIOMEDICAL RESEARCH

This Committee and Congress are to be commended for their support for biomedical research through the years. It is vital that we continue to sufficiently fund the NIH, so that it can capitalize on scientific advances and maintain the momentum generated by the doubling of funds and the infusion from the American Recovery and Reinvestment Act (ARRA). These increases in funding brought a new era in drug discovery that has benefited all Americans.

Cutting discretionary health spending by 13.5 percent, as has been proposed, would halt this progress. Deep cuts would have a detrimental effect on the fight against many of our most serious diseases, stifle scientific opportunities, and result in high-wage job loss in all 50 states. In 2007, NIH grants and contracts created and supported more than 350,000 jobs across the United States, an important contribution to the American economy.

We urge this Committee and Congress to maintain robust investment in biomedical research at the NIH so it can fund critical research today that will provide the care and cures of tomorrow.

STRENGTHENING CLINICAL RESEARCH AND DRUG DEVELOPMENT

The Cystic Fibrosis Foundation has been recognized for its unique research approach, which encompasses everything from basic research through Phase 4 post-marketing monitoring of drug safety, 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 30 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 CFF. This promising therapy targets the physiological defect that causes CF in patients with a particular type of genetic mutation, as opposed to only addressing symptoms of the disease. In late February 2011 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.

The Foundation is a leader in creating a clinical trials network to achieve greater efficiency in clinical investigation. Because the CF population is small, a higher proportion of people with the disease must partake in clinical trials than in most other diseases. This unique challenge prompted the Foundation to streamline our clinical trials processes. As a result, research conducted by the Foundation is more efficient than ever before and we are a model for other disease groups.

While the CF Foundation has made great progress in creating a more efficient drug development process for cystic fibrosis, still more needs to be done for other rare diseases, many of which have no treatments available. The federal government has the opportunity to make a real difference in this regard, and we are hopeful that the Committee will direct the national health agencies to encourage all investigators and institutions receiving federal funding to advance novel methodologies and mechanisms for translating basic research into therapies that can benefit patients.

Advancing Translational Science

The CF Foundation strongly urges this Committee and Congress to support funding for NIH's proposed National Center for Advancing Translational Sciences (NCATS), which will house the Institutes' existing translational science programs while establishing and providing a more focused, integrated, and systematic approach for linking basic discovery to therapeutic development.

The existing programs to be housed under NCATS are integral to translating basic science into treatments and will benefit from funding for the new center. These programs include Clinical and Translational Science Awards (CTSA), discussed in further detail below, and the newly authorized Cures Acceleration Network (CAN), both designed to transform the way in which clinical and translational research is conducted and funded. The Therapeutics for Rare and Neglected Diseases (TRND) program will also be

housed in the new center. NIH Director Collins has specifically cited the Cystic Fibrosis Foundation's Therapeutics Development Network (TDN), which plays a pivotal role in accelerating the development of new treatments for cystic fibrosis patients, as an exemplar for TRND's innovative therapeutics development model.

The Foundation's investment in pharmaceutical and biotech companies can also serve as a model for the new center's overall mission. NCATS, like CFF, will promote public-private partnerships and convene cross-sector collaborations between industry, government, academia, and others to advance drug development, as well as provide services and resources for high throughput screening, assay development, and preclinical modeling. Prioritizing these initiatives through a standalone center at NIH has the potential to greatly accelerate the development of drugs for diseases that have historically received little pharmaceutical industry attention. In addition, integrating translational science programs from throughout NIH into one center will help bring greater efficiency to the Institutes' pursuit of this important research. Once again, we applaud NIH Director Collins for spearheading NCATS and look forward to working with him as this new initiative is implemented.

Clinical and Translational Science Awards (CTSA)

The CTSA program, soon to be housed in NCATS, encourages novel approaches to clinical and translational research, enhances the utilization of informatics, and strengthens the training of young investigators. Key to the success of CTSA is the parallel maintenance of infrastructure support for Clinical Research Centers (CRC). Without a mechanism to offset clinical research costs, young investigators or Principle Investigators (PIs) studying rare diseases for which there is limited funding will not be able to continue to conduct clinical research. It is important that all NIH institutes recognize that there is a significant cost associated with the conduct of well designed and safe clinical trials, and not all of these costs can be borne by the CTSA. Congress should direct the NIH to cover costs that used to be borne by the General Clinical Research Centers (GCRCs) through individual research grants.

Support should also be directed toward the continuation and expansion of research networks, such as NIH's pediatric liver disease consortium at the National Institute of Diabetes, Digestive, and Kidney Diseases (NIDDK). This successful collaboration is helping researchers discover treatments not only for CF liver disease but for other diseases that affect thousands of children each year.

SUPPORTING DRUG DISCOVERY

The Cystic Fibrosis Foundation's clinical research is fueled by a vigorous drug discovery effort comprised of early stage translational research into successful treatments for this disease. Several research projects at the NIH will expand our knowledge about the disease, and could eventually be the key to controlling or curing cystic fibrosis.

Opportunities in Animal Models

The Cystic Fibrosis Foundation is encouraged by the NIH's investment in a research program at the University of Iowa to study the effects of CF in a pig model. The program, funded through research awards from both the National Heart, Lung, and Blood

Institute (NHLBI) and the Cystic Fibrosis Foundation, bears great promise to help make significant developments in the search for a cure. While a company has been established to produce the animals, the infrastructure and extensive animal husbandry required to keep the animals alive and conduct research on them is available at few academic institutions. Such barriers have greatly limited widespread adoption of these valuable research tools. We urge additional funding to create a common facility that would enable researchers from multiple institutions to conduct research with these models.

Understanding CFTR Folding and Trafficking

The data that emerged from the VX-770 Phase 2 and 3 clinical trials, discussed above, is proof that the way in which this drug targets the physiological defect that causes CF, called CFTR protein function modulation, is a viable therapeutic approach. However, this exciting data was obtained from patients with a specific CF mutation which affects only approximately 4 percent of CF patients. More research is needed to understand other genetic mutations, the most common of which is called F508del. F508del causes multiple negative effects, including misfolding and poor activation properties of the CFTR protein. We encourage the Committee to increase investment in genetic research that can help scientists to better understand the F508del mutation. This will facilitate CF drug discovery and has the potential to benefit not just those with cystic fibrosis, but also those with other protein misfolding diseases.

Personalized Medicine

Strong federal and private investment in research is bringing personalized medicine into the forefront. As we gain a deeper understanding of many diseases and their accompanying genetic profiles, we understand the great challenge of personalizing therapies. While exciting and promising for patients, it is also expensive, complex, and scientifically challenging. For instance, CF doctors are facing difficulties in delivering appropriate care to CF patients, as insurance providers will not cover certain combinations of medicines that clinicians have found are effective for cystic fibrosis in particular when there is no formal clinical data to support it. This puts patients in a difficult position, as these clinical trials are expensive and unlikely to be performed by pharmaceutical companies, especially for treatment of a small, targeted population. As such we urge the Committee to provide sustained federal investment in personalized medicine, to help move this burgeoning field forward and advance exciting scientific discoveries.

SUPPORTING GREATER ACCESS TO QUALITY HEALTH CARE

We are making remarkable strides in our fight against cystic fibrosis, but people who live with it face greater obstacles each year, as high medical costs can prevent them from accessing appropriate medical care. Health care for a CF patient costs \$64,000 per year on average, 15 times more than that of the average person. Because of high costs, nearly a quarter of CF patients delay getting medical care or skip treatments their providers recommend to enhance and lengthen their life.

The Foundation sees some promise in a number of provisions in the new health care reform law that increase access to health insurance coverage for those with rare and chronic diseases, a critical tool in decreasing out of pocket costs for patients. These provisions include those allowing children to remain on their parents' insurance until they are 26; prohibiting insurance companies from denying or rescinding coverage based on a pre-existing condition; banning annual and lifetime caps on coverage; and the expansion of Medicaid eligibility.

The new law is not perfect, however, and we are concerned that while the provisions listed above will ensure continuity of coverage and greater access to care for those with CF and other chronic diseases, more must be done to reduce the financial burden so many families face in affording their care, especially in these challenging economic times.

While we urge Congress to explore new options to help make care more affordable and reduce shifting costs to patients, we ask that provisions that have the potential to provide desperately needed relief to people with cystic fibrosis be retained, and that they are sufficiently funded so that those with rare and chronic diseases can access the care they need.

In addition, the Foundation wishes to applaud the formation of the Patient Centered Outcomes Research Institute (PCORI) and urges the Committee to support this important entity. PCORI, a private non-profit institute created by the Patient Protection and Affordable Care Act, will support and direct research that gives patients, doctors, and others the information they need make informed decisions about the most effective and appropriate methods for preventing and treating health conditions. The CF Foundation has had great success in improving quality of care for cystic fibrosis patients through the development and administration of a comprehensive patient registry and the collection of comprehensive data on outcomes and practice patterns for use in comparative effectiveness research, and we are confident that dedicating a national institute to such pursuits will improve care for all Americans.

The Cystic Fibrosis Foundation has devoted our own resources to developing treatments through drug discovery, clinical development, and clinical care. Several of the drugs in our pipeline show remarkable promise in clinical trials and we are increasingly hopeful that these discoveries will bring us even closer to a cure. However, sufficient investment in basic science, translational science, clinical research, and drug development programs at NIH is needed to continue these successes not only for CF but for all rare diseases. Additionally, funding for programs that promote access and quality of care will help achieve a greater quality of life for those living with chronic diseases like cystic fibrosis.

We urge the Committee to consider these factors as you craft the Fiscal Year 2012 Labor, Health and Human Services, and Education Appropriations legislation, and stand ready to work with NIH and Congressional leaders on the challenging issues ahead. Thank you for your consideration.