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Testimony Prepared for the House Appropriations Subcommittee on Labor, Health and Human Services, Education, and Related Agencies
Topic: Requesting funding for the National Institutes of Health, Centers for Disease Control, and the Health Resources and Services Administration in FY 2019

On behalf of the Cystic Fibrosis Foundation and the approximately 30,000 people with cystic fibrosis (CF) in the United States, we submit the following testimony to the House Appropriations Committee’s Subcommittee on Labor, Health and Human Services, Education, and Related Agencies on our funding requests for Fiscal Year 2019. We appreciate the successful bipartisan effort by Congress earlier this year to raise the budget caps and hope these higher numbers will allow the Committee to prioritize funding for the vital health programs described below. In particular, the Cystic Fibrosis Foundation requests:

- $39.3 billion in funding for the National Institutes of Health (NIH) to support basic, translational, and clinical science as well as development of the next generation of researchers;
- $15.65 million for the Centers for Disease Control and Prevention’s (CDC) newborn screening program, in addition to increased support for the CDC’s flu activities and antimicrobial resistance activities; and
- $19.9 million for the Health Resources and Services Administration’s (HRSA) heritable disorders program, a $2 million increase for the Division of Transplantation, and increased support for HRSA’s newborn screening program.

National Institutes of Health
NIH Supports Advances in CF through Cost-Efficient, Collaborative Research

As the Committee considers its funding priorities for the coming fiscal year, we urge consideration of the critical role that NIH plays in improving the lives of patients with cystic fibrosis and other rare diseases. Cystic fibrosis is a rare genetic disease that causes the body to produce thick mucus that clogs the lungs and other bodily systems, resulting in life-threatening infections, diabetes, malnutrition, and other medical complications. Incredible progress has been made in CF care and drug development over the last five decades. In the 1950’s, children with cystic fibrosis did not live to attend elementary school. Today people with CF are living into their 30’s, 40’s, and beyond. These advancements would not have been possible without the research supported by the NIH, and we request a funding level of at least $39.3 billion for NIH in FY19.

According to the NIH’s RePORT system, NIH devoted $91 million to cystic fibrosis research in fiscal year 2017, and a strong funding partnership between NIH and the Cystic Fibrosis Foundation has enabled additional groundbreaking research and advances. The CF Foundation collaborates with the NIH to fund and organize initiatives at all stages of scientific investigation from basic and translational research to advancing new CF therapies to evaluation of existing methods of CF care and treatment. Providing funding for the NIH is an effective way to foster collaboration with external stakeholders, advance new treatments for CF, and apply lessons learned from CF drug development to bring new directions to research for other common disorders such as chronic obstructive pulmonary disease (COPD), pancreatic disorders, and infertility.
NIH Supports Vital Basic Research

Basic research funded by the NIH helps builds foundational knowledge in cellular and molecular processes to help us improve our knowledge of the underlying cause and progression of diseases like CF. For example, researchers funded by the NIH and CFF at the University of Alabama Birmingham and Columbia University are using cryo-electron microscopy to better understand the structure and function of the cystic fibrosis transmembrane regulator (CFTR) proteins inside the body. Work like this is critical to understanding the underlying cause of CF and may lead in the future to new targeted treatments for this devastating rare disease.

NIH and CFF are also collaborating to tackle basic research on some of the most complex barriers to advancing gene editing technology as a CF therapy. Use of these new tools is especially difficult in cystic fibrosis because the buildup of sticky mucus in the lungs of those with CF can prevent delivery of potential gene editing treatments through traditional methods. Earlier this year the National Heart Lung and Blood Institute held a joint workshop with the CF Foundation to convene researchers for a discussion on the development and evaluation of viable gene delivery technologies in those with CF, and promising research is ongoing in this area.

Advancing Translational Science

NIH funding for translational research tools supports the development of new therapies for rare diseases like cystic fibrosis. Between 2010 and 2016, NIH supported research that contributed to 210 new FDA-approved drugs, vaccines, and new indications for current drugs. To continue this important work, the Foundation requests robust funding for NIH’s National Center for Advancing Translational Sciences (NCATS), which catalyzes innovation by improving the diagnostics and therapeutics development process and removing obstacles to translating basic scientific research into treatments.

The specific programs housed in NCATS are integral to this mission, including the Clinical and Translational Science Awards (CTSA), the Cures Acceleration Network (CAN), and the Therapeutics for Rare and Neglected Diseases (TRND) program. Such initiatives transform the way in which clinical and translational research is conducted and funded. NIH Director Dr. Francis Collins has cited the CF Foundation supported Therapeutics Development Network (TDN), a CF-dedicated clinical trials network, as a model for TRND’s innovative therapeutics development model.

The Foundation also urges additional funding for the Cystic Fibrosis Research & Translation Centers (CFRTCs), which provide support for basic, preclinical, and clinical research efforts to advance scientific knowledge and new therapies for CF at seven centers across the country. CFRTCs are cost-efficient, providing shared resources and facilities to enhance collaboration and multi-disciplinary work in cystic fibrosis. NIDDK provides funding for the CFRTCs through P30 Center Core grants, which the CF Foundation is able to further support by providing grants for individual CF researchers at the Centers. Funding increases at NIH in recent fiscal years have provided critical support to these programs, and momentum must continue so large centers can continue research programs and maintain their infrastructure and promote funding certainty for small-operation CF research programs, which play an instrumental role in recruiting new investigators into CF research.

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Animal models are also an important, NIH-supported tool for understanding disease progression and identifying potential new treatments for CF and other rare diseases. The National Swine Resource and Research Center (NSRRC), funded by the NIH and hosted at the University of Missouri-Columbia, provides services to develop swine models of many genetic conditions, like cystic fibrosis, in order to facilitate research and drug development for these diseases. NIH and the CF Foundation also jointly fund a research program at the University of Iowa to study the effects of CF in a ferret model, and the University of Alabama at Birmingham has used joint funding to develop multiple CF rat models to examine methods for studying basic mechanisms and treatment of the disease. These programs are yielding fundamental new insights to help advance developments in the search for life-changing treatments for CF.

**Improving Clinical Care**

Research in dissemination and implementation science that focuses on integrating scientific findings and effective clinical practice into real-world settings is crucial to providing the best possible care to those with CF and other conditions. NIH also provides support for advancing optimal care and treatment use for those with CF. The OPTIMIZE study, which receives joint funding from the NIH and the CF Foundation, has brought together hospital systems in nearly 30 states to compare the effectiveness of combining antibiotic treatments for lung infections in those with cystic fibrosis. Findings from this initiative could help advance quality care for those with CF and improve our understanding of effective use of these therapies in specialized CF care centers.

**Supporting the Next Generation of Researchers**

We strongly urge the Committee to provide robust resources for the NIH to address challenges in recruiting and retaining a strong scientific workforce. It is difficult to recruit scientists into rare disease research, especially in pediatric subspecialties. Robust funding for programs like the K awards, which support early-career investigators, are critical to attracting and retaining a strong scientific workforce. Supporting junior investigators, especially those who specialize in rare diseases and pediatric subspecialties is a crucial element in the fight to find a cure for CF and countless other diseases for which there are not adequate treatment options.

**Consistent, Robust Funding for NIH is Critical for American Research**

We appreciate the $3 billion funding increase provided to NIH in Fiscal Year 2018. However, NIH has not yet overcome the devastating and lasting effects of many years of sequestration and stagnant funding on American research labs both at intramural and extramural research institutions. Funding success rates for all investigators remain below sustainable levels, and promising young investigators struggle to obtain sufficient funding to remain in their respective fields. Recent increases in funding have helped to mitigate the after effects of stagnant funding, but this growth must continue.

Further, NIH is an important driver of the US economy, providing over 400,000 jobs and nearly $69 billion of economic output in Fiscal Year 2017. Increased investment in biomedical research can provide even greater economic benefit and support for the scientific progress that makes the United States the international leader in biomedical research.

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Centers for Disease Control and Prevention

The Centers for Disease Control and Prevention (CDC) plays an important role in helping individuals with CF live longer, healthier lives. Particularly, we ask you to give special consideration to CDC’s role in the facilitation of newborn screening to detect congenital disorders, in addition to the CDC’s work on antibiotic resistance and flu.

In 2016, 62.4 percent of new CF diagnoses were detected through newborn screening, and there is evidence that individuals diagnosed early-on, prior to the onset of symptoms, have better lung function and nutritional outcomes later in life. The earlier a child is diagnosed with CF, the sooner their families and clinicians can develop a treatment plan that includes airway clearance techniques, nutritional therapies and medicines that may significantly reduce cumulative damage caused by the disease. Funding for newborn screening programs from this committee has done a tremendous amount for state-based programs. However, more can be done to improve this critical public health function.

In particular, the Foundation urges the Committee to provide $15.65 million (an increase of $6 million) in funding to the CDC’s newborn screening program, which is responsible for strengthening and enhancing laboratory quality assurance programs; enabling public health laboratories to develop and refine screening tests; conducting pilot studies; implementing new methods to improve detection of treatable disorders; and enhancing newborn disorder detection through the Newborn Screening Quality Assurance Program.

The CF Foundation also calls upon the Committee to further support the efforts of the CDC in combating antimicrobial resistance. People with CF are subject to frequent and chronic lung infections, which are the leading cause of morbidity and mortality for the disease. To combat chronic lung infections, many people with CF take antibiotics as part of their daily treatment regimen. Because people with CF are more susceptible to lung infections, the upsurge of antibiotic resistance is of the utmost concern. The work of the CDC to prevent the spread of antibiotic resistant organisms through improving antibiotic prescribing and stewardship, tracking resistance patterns, promoting immunization, and developing new antibiotics is critical in maintaining the health of those with CF. Through a broad agency announcement, the CDC is also funding a project examining how to optimize therapeutic strategies to manage polymicrobial CF lung infections. We hope the Committee will prioritize funding for CDC’s activities so this and other important work can continue in FY19.

Additionally, the CDC plays an important role in protecting the safety of the public through controlling and preventing infectious diseases. For example, the CDC is a key player in the development and nationwide distribution of flu vaccinations as well as in flu surveillance. People with CF are especially susceptible to contracting the flu and, in some cases, the virus can become life-threatening and lead to lengthy hospital stays. Because of the severity of the flu in the CF community, we appreciate the collaborative work of the Department of Health and Human Services, including at NIH, CDC, ASPR and FDA to prepare for and seek to minimize the morbidity and mortality of the flu virus every year. It is also imperative that HHS receives the funding necessary to develop a more effective and modern universal flu vaccine.

Health Resources and Services Administration

We also encourage the Committee to provide $19.9 million (an increase of $6 million) for HRSA’s heritable disorders program, which evaluates the effectiveness of newborn screening
and follow-up programs and provides grants for programs to support other critical aspects of newborn screening. Additionally, within HRSA, we encourage strong support for the Title V Maternal and Child Health Services Block Grants program, which provides flexible funding for states to support programs that provide access to quality care for low-income and underserved people and create systems of coordinated care for children with special health care needs. In many states, these grants enable the provision of comprehensive newborn screening education, services, and follow up.

Additionally, the CF Foundation appreciates the $2 million increase in funding for the Division of Transplantation within HRSA in Fiscal Year 2018 and urges the Committee to continue robust funding for the program in FY 2019. Cystic fibrosis is a degenerative disease that can cause severe damage and ultimately failure of the lungs. Those with CF who experience extensive lung damage may consider transplant as a way to regain critical lung function and continue living full, productive lives. In 2016, 1,642 individuals in the CF patient registry identified as receiving a lung, kidney, heart, or liver transplant with an additional 151 individuals who are approved candidates for transplant but are on the waiting list.

The oversight HRSA provides to the transplant network through operation of the United Network for Organ Sharing (UNOS) is crucial in promoting the safety and efficacy of organ transplantation. In recent years, the CF Foundation has seen a marked increase in the need for donor lungs in our patient community. To address this need, we created a lung transplant initiative in 2016 which offers education and support services for CF patients seeking a lung transplant. However, we believe that permanent changes to the geographic allocation of donor lungs are needed to deliver lungs to the patients who need transplants the most. To support this and other critical work at UNOS, we ask the Committee to provide robust funding for the Division of Transplantation in Fiscal Year 2019.

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This is a time of great hope and optimism for the CF community and those with other rare diseases, as more research is being conducted to treat these life-threatening conditions. We urge you to provide at least $39.3 billion for the National Institutes of Health as well as robust funding for other relevant agencies to support health care quality research and newborn screening. We stand ready to work with the Committee and Congressional leaders on the challenges ahead. Thank you for your consideration.

Sincerely,

Preston W. Campbell, III, MD
President and Chief Executive Officer

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