



May 23, 2019

The Honorable Nita Lowey  
Chairwoman  
House Appropriations Committee  
US House of Representatives  
Washington, DC 20515

The Honorable Richard Shelby  
Chairman  
Senate Appropriations Committee  
US Senate  
Washington, DC 20510

The Honorable Kay Granger  
Ranking Member  
House Appropriations Committee  
US House of Representatives  
Washington, DC 20515

The Honorable Patrick Leahy  
Ranking Member  
Senate Appropriations Committee  
US Senate  
Washington, DC 20510

Dear Chairwoman Lowey, Chairman Shelby, and Ranking Members Granger and Leahy:

On behalf of the Cystic Fibrosis Foundation, I am writing to express our support for robust funding for the National Institutes of Health (NIH) and the Food and Drug Administration (FDA) for Fiscal Year 2020. We appreciate the successful bipartisan effort by Congress to raise funding for both the NIH and the FDA for FY 2019, and we were pleased to see the House demonstrate its continued commitment to strong NIH funding with the inclusion of \$41.1 billion for the Institutes in the Labor-HHS-Education FY 2020 funding bill. The CF Foundation hopes to see this support extend to both agencies as Congress moves forward with FY 2020 appropriations. The ongoing work at these agencies remains critical to advancing CF research and drug development and bringing us closer to a cure for this devastating disease.

As you consider appropriations for federal agencies for fiscal year 2020, the Cystic Fibrosis Foundation requests that Congress provides the following:

- \$41.6 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; and
- \$3.4 billion in funding for the Food and Drug Administration (FDA), including a \$316 million increase in medical product program funding, to enable the agency to keep pace with scientific advances and to expeditiously review life-changing therapies while maintaining the highest regulatory standards.

The Cystic Fibrosis Foundation is a national organization dedicated to curing cystic fibrosis (CF) – a chronic, life-threatening genetic disease that affects over 30,000 people in the United States. Our mission is to provide all people with the disease the opportunity to lead full, productive lives by funding research and drug development, promoting individualized treatment, and ensuring access to high-quality, specialized care.

We have made great progress in CF care and drug development over the last five decades. At the time of the Foundation's creation in 1955, children with cystic fibrosis rarely lived to attend

elementary school. Today, thanks to the advances in understanding of the disease and the development of CF-specific therapies, people with CF are living into their 30's, 40's, and beyond. There are currently 13 FDA-approved therapies that treat the underlying cause of CF or complications caused by the disease, some of which were reviewed in record-breaking time. These therapeutic advances have been pivotal for improving outcomes for people with CF.

We are reaching a momentous time in the CF community; we anticipate that with the likely introduction of a new drug that treats the underlying cause of the disease by as early as 2020, more than 90 percent of people with CF will eventually have a highly effective treatment available for their genetic mutation. While not a cure, this drug – called the triple combination modulator – is expected to drastically improve symptoms and slow the progression of the disease.

### **National Institutes of Health**

#### **NIH Supports Vital Basic Research**

As Congress considers its funding priorities for the coming fiscal year, we urge consideration of the important role that NIH plays in improving the lives of patients with cystic fibrosis and other rare diseases. The NIH spent \$96 million on CF-related research in fiscal year 2018, which helped support basic research that builds foundational knowledge in cellular and molecular processes and improves understanding of potentially transformative therapies and technologies for CF. The basic science research that NIH supports could lead to tomorrow's therapies, and eventually a cure for this devastating disease.

#### **Infection**

Infection is a critical concern in the CF community. The buildup of thick, sticky mucus in the lungs characteristic of the disease makes people with cystic fibrosis more prone to bacterial infections. Many people with CF suffer from difficult-to-treat acute or chronic infections, which can become life-threatening if not treated adequately. NIH-supported basic science research on infections as well as research evaluating the efficacy and use of available antibiotics must be prioritized to address the growing threat of resistant infections in vulnerable populations. 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. The NIH is a partner in our efforts to find solutions to challenges people in the CF community face like difficult-to-treat infections, and strong funding is needed to continue these collaborative efforts.

#### **Gene Editing and Gene Therapy**

NIH funding supports important basic science research advancing the fields of gene therapy and gene editing. While we expect the approval of an effective therapy that treats the underlying cause of the disease for a significant percentage of the population, it is not a cure. And at least 7 percent of the CF population will not be able to benefit from these therapies. Gene editing and gene therapy will likely play an important role in the future of CF therapies, especially as we search for effective treatment options for those who will not have access to a modulator drug.

The NIH plays an important role in advancing new technologies like gene editing, especially in diseases where complex barriers stand in the way of applying those technologies. For example, gene editing for the treatment of cystic fibrosis may be challenging due to the difficulty of effectively delivering gene therapies into the lungs. In 2018, the National Heart Lung and Blood

Institute held a joint workshop with the CF Foundation. This event convened researchers for a discussion on the development and evaluation of viable gene delivery technologies for treating CF to help overcome obstacles for therapeutic development in this area. Additionally, NIH launched a Somatic Cell Genome Editing research program funding researchers aimed at developing tools for safe and effective genome editing in disease-relevant cells and tissues for rare diseases including CF.

### **NIH Supports the Next Generation of Researchers**

Additional resources are needed for the NIH to continue 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, is vital for 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.

### **Food and Drug Administration**

The FDA performs essential work to ensure that drugs that make it into the hands of patients are both safe and effective. A well-funded FDA ensures drugs are reviewed adequately and that the agency is able to perform reviews of new products in a timely manner. Without sufficient funding for the FDA, there could be unnecessary delays in the approval of life-saving drugs. Increased funding will also help regulators keep pace with scientific advances that underlie the numerous innovative products in the drug development pipeline.

### **FDA Staffing is Integral for a Timely and Thorough Product Review Process**

The agency's most valuable resource is its staff; ensuring the FDA is able to compete with the private sector is critical to securing the appropriate expertise in-house for medical products review. This is a particularly acute issue for rare disease products, where having the appropriate expertise is necessary for understanding the nuances of the disease as well as the unique challenges experienced by the patient community. Since 2012, the FDA was able to review three drugs that target the underlying defect in CF, including some in record time. This would not have been possible without the in-house expertise the agency has been able to cultivate.

### **Advancing innovative clinical trials design**

Rare disease communities often face unique challenges such as small patient populations that make it more difficult to carry out traditional clinical trials. The agency's acceptance of more innovative clinical trial designs has been critical to removing some of the barriers rare disease communities face during the drug development process. It is important that the FDA has the resources available to continue their work in advancing the use of innovative clinical trial designs to help further rare disease drug development where traditional clinical trial designs may not be feasible.

The FDA's Complex Innovative Trial Designs (CID) Pilot Meeting Program invites drug developers to apply for assistance in employing novel trial designs during clinical studies. Increasing the ways in which drug developers can interact with the agency is critical given that developing successful innovative designs requires more agency input to ensure that studies are carried out effectively and ethically.

## Regulatory Flexibility for Rare Diseases

Regulatory flexibility has played an important role in evaluating breakthrough therapies for rare diseases. In 2017, the FDA developed a novel approach to expand the use of Kalydeco – a modulator drug – for certain rare CF mutations. This approach allowed the FDA to use laboratory data when a full clinical trial was not possible. It is critical that the FDA has sufficient capacity to develop the internal expertise and guidance for industry that will enable continued innovations such as this.

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This is a time of great hope and optimism for the CF community, but we are not yet done. The NIH and FDA perform vital work that supports the development of novel therapies from inception to end product, and strong funding in this upcoming fiscal year will be needed to continue their valuable efforts. We stand ready to work with the Committee and Congressional leaders on the challenges ahead. Thank you for your consideration.

Sincerely,



**Mary B. Dwight**  
Senior VP of Policy & Advocacy  
Cystic Fibrosis Foundation

cc: The Honorable Rosa DeLauro  
The Honorable Tom Cole  
The Honorable Sanford Bishop Jr  
The Honorable Jeff Fortenberry

The Honorable Roy Blunt  
The Honorable Patty Murray  
The Honorable John Hoeven  
The Honorable Jeff Merkley