Organization Submitting Testimony: Cystic Fibrosis Foundation

On behalf of the Cystic Fibrosis Foundation (CFF) and the 30,000 people with cystic fibrosis (CF) in the United States, we are grateful for the opportunity to submit the following testimony for consideration by the Senate Appropriations Committee for its hearing, "Driving Innovation through Federal Investments." The Foundation applauds the Committee for facilitating a dialogue about this critical issue.

Thanks to federal investment in innovative research and development, treatments for cystic fibrosis and other rare, chronic diseases are changing the lives of millions of patients. This testimony will discuss how collaborative, innovative ventures administered by NIH effectively and efficiently spur drug development and exemplify what can be achieved when government funding is leveraged on behalf of patients.

Collaboration and Innovation: The Future of Drug Development

Developing treatments is expensive and time consuming, with a 95 percent failure rate on average for drug candidates. NIH administers programs that promote efficiency and innovation in drug discovery, to help accelerate this process and reduce the time, cost, and risk of drug development by encouraging stakeholders to share resources, data, best practices, and expertise. This approach can help move treatments more quickly to patients who live every day with serious, life-threatening illnesses.

As an example of the NIH's cooperative, innovative approach, in February the agency announced the establishment of the Accelerating Medicines Partnership (AMP), a joint venture between NIH, pharmaceutical companies, and several non-profit organizations to characterize biomarkers and distinguish biological targets that are most likely to respond to new therapies. The AMP will begin with three to five year pilot projects in Alzheimer's disease, type 2 diabetes, rheumatoid arthritis and systemic lupus erythematosus.

Through this cross-sector partnership, NIH and industry partners share expertise, resources, and data in order to speed the development of treatments. Industry partners have agreed to make AMP data and analyses available to the biomedical community for use in future study, so they can be analyzed in ways that drug companies have not been able to do on their own.

Importantly, industry will fund one-half of the $230 million budget while NIH will provide the other half. The federal money used for this project acts as seed money, a jumping off

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point for private sector investment in drug discovery for serious diseases. This type of cooperative approach saves taxpayer funds in the long run and can save lives.

The National Center for Advancing Translational Sciences (NCATS) spearheads similarly innovative programs that encourage collaboration, improve the process by which diagnostics and therapeutics are developed, and improve the efficiency of the translation of basic scientific discoveries into new therapies. For example, the Clinical and Translational Science Awards (CTSA) program exemplifies an innovative, collaborative approach. This program supports a national consortium of more than 60 medical research institutions that share best practices and work together on research. Its goals are to accelerate the process of translating laboratory discoveries into treatments for patients, train a new generation of researchers, and engage communities in clinical research efforts.

The Cystic Fibrosis Foundation has long engaged in partnerships with industry and supports a collaborative network of care centers and clinical trial sites. As such, CFF knows firsthand that this type of cooperation can lead to the targeted treatments that change the face of life-threatening diseases.

Because drug research and development is a lengthy, expensive and risky process, CFF pioneered a successful "venture philanthropy" business model to drive drug development for this rare disease. By collaborating with pharmaceutical companies and providing financial, scientific, and clinical support in order to "de-risk" the development process, CFF speeds development of much-needed treatments.

Through its venture philanthropy model, the Foundation is able to invest in promising CF research and a robust pipeline of potential therapies that target the disease from every angle. Nearly every CF drug available today was made possible because of the Foundation's support and ongoing work with researchers and the pharmaceutical industry to find a cure.

In January 2012, the Food and Drug Administration (FDA) approved Kalydeco, a groundbreaking cystic fibrosis drug developed by Vertex Pharmaceuticals in partnership with the CF Foundation. This targeted drug is the first to address the underlying genetic cause of cystic fibrosis in a subset of the CF population.

Kalydeco was approved in only 3 months, one of the fastest approvals in the FDA's history. According to Margaret A. Hamburg, M.D., Commissioner of the FDA, "The unique and mutually beneficial partnership that led to the approval of Kalydeco serves as a great model for what companies and patient groups can achieve if they collaborate on drug development."

Throughout Kalydeco's review, the Cystic Fibrosis Foundation and renowned CF experts worked closely with Vertex Pharmaceuticals and the FDA, providing valuable insight on specific issues related to CF, clinical research on CF treatments, and other issues related to the product and its review. We believe that this collaborative process contributed to a more efficient evaluation, and is a testament to what can be achieved when stakeholders collaborate across sectors on critical drugs for patients.

Importantly, NIH-funded advances, like the development of high-throughput screening, were essential to the creation of Kalydeco, and more NIH-funded research is underway to understand the more than 1,000 other genetic mutations of CF. These NIH-funded studies, often funded in partnership with the CF Foundation, are critical to finding a cure for CF in our lifetime.

Investment in innovative programs at NIH is important to the health and well-being of Americans, but it is also important to the health of our economy. NIH funding supported more than 402,000 jobs and $57 billion in economic output in 2012, according to a report by United for Medical Research. Robust funding for NIH promotes much-needed economic growth and supports the scientific progress that makes the United States the worldwide leader in biomedical research.

Unfortunately, budget sequestration in recent years is hampering American innovation, and the CF Foundation is concerned about the impact of further sequestration of NIH funds in the coming years. For example, the American Society for Biochemistry and Molecular Biology estimates that the budget cuts brought about by sequestration resulted in 1,001 fewer investigators who had NIH funding through standard research mechanisms in 2013. The year prior, the number of funded investigators had dropped by just 150.

There is evidence that these cuts are discouraging the next generation of scientists from engaging in scientific research. In a recent survey conducted by the Association of American Medical Colleges, nearly two-thirds of the Ph.D. programs that responded reported that they have decreased or are anticipating decreasing the size of their programs. Nearly one in five M.D.-Ph.D. programs indicated a decrease or anticipated decrease in students in the coming year. It is clear that recent budget cuts are having an impact.

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Once again, the Cystic Fibrosis Foundation commends the Committee for its evaluation of the benefits of government investment in innovation. As we move into an era of personalized medicine, it is critical that our research agencies nurture and grow the collaborative ventures that help make the U.S. the leader in biomedical research.

The CF Foundation stands ready to work with the Committee, NIH, and Congressional leaders on the challenges ahead. Thank you for your consideration.