



February 4<sup>th</sup>, 2022

The Honorable Patty Murray  
Chair, U.S. Senate Committee on  
Health, Education, Labor and Pensions  
428 Senate Dirksen Office Building  
Washington, D.C. 20510

The Honorable Richard Burr  
Ranking Member, U.S. Senate Committee on  
Health, Education, Labor and Pensions  
648 Hart Senate Office Building  
Washington, D.C. 20510

Re: Prepare for and Respond to Existing Viruses, Emerging New Threats, and Pandemics Act (PREVENT Pandemics Act) Discussion Draft

Dear Chair Murray and Ranking Member Burr:

On behalf of the Cystic Fibrosis Foundation, I write to provide comments on the discussion draft of the Prepare for and Respond to Existing Viruses, Emerging New Threats, and Pandemics Act (PREVENT Pandemics Act). We appreciate the committee's dedication to ensuring that the United States will have the infrastructure and resources necessary to combat future public health crises.

### **Background on cystic fibrosis and COVID-19**

The Cystic Fibrosis Foundation is a national organization actively engaged in the research and development of new therapies for cystic fibrosis – a rare, life-threatening genetic disease that affects more than 30,000 people in the United States. People with CF face a heightened life-long risk of both viral and bacterial infections because of the thick, sticky mucus in the lungs that is characteristic of the disease. Unfortunately, infections can lead to losses in lung function, and many people with CF battle difficult-to-treat infections for which there are no effective antimicrobial drugs available. Additionally, like many other patient populations with underlying conditions, people with CF can face increased risk for adverse outcomes from COVID-19 infection.

The impacts of the COVID-19 pandemic on the CF community have been wide-ranging. The pandemic has affected the CF community's ability to safely engage in necessary activities, including accessing routine specialty care and attending school. Due to the increased risk from infection, people with CF have experienced increased rates of depression and anxiety during the pandemic. And perhaps most notably, people with CF who are post-transplant are especially vulnerable and have not been able to benefit from the protection offered by vaccines.

While we appreciate all of the important steps outlined in this legislation to prepare for the next pandemic, it is also critical that the legislation address issues specific to individuals with chronic conditions and the unique challenges they face, as we describe below.

**Sec. 112. Supporting access to mental health and substance use disorder services during public health emergencies.**

People with CF and their caregivers experience anxiety and depression at higher rates than the general population, a burden that was exacerbated by the stress of the COVID-19 pandemic. It is therefore encouraging that the committee recognizes how critical mental health and substance use disorder services are during public health crises. We suggest that the committee devote attention not just to supporting continued access to mental health and substance abuse disorders during public health crises, but also increasing access to them—both through expansion of the services themselves and subsequent dissemination of information regarding their availability.

**Sec. 201. Addressing social determinants of health and improving health outcomes. & Sec. 202 National Academies of Sciences report**

We are pleased to see the PREVENT Pandemics Act devotes considerable attention to studying and reducing health and healthcare disparities, addressing social determinants of health, and partnering with local organizations to meet the needs of underserved populations. These topics are of considerable importance to the Cystic Fibrosis Foundation: people of color with cystic fibrosis are underrepresented in clinical trials, are diagnosed later in life, and have poorer outcomes compared to their white counterparts. We would encourage collaboration and consultation with government agencies that have ongoing work in this field, including the National Institute on Minority Health and Health Disparities and the Patient Centered Outcomes Research Institute, both to leverage their expertise and to avoid inefficiency through duplicative efforts. We appreciate that applicants are required to base their activities on a community needs assessment and incorporate community input. To ensure long-term impact, scalability and sustainability should also be considered as grant criteria. Finally, we appreciate the request for the National Academies of Sciences to write a report on these important topics.

**Sec. 212. Genomic sequencing, analytics, and public health surveillance of pathogens.**

The tools developed and research performed under Sec. 212 are intended for the purpose of public health surveillance, preparedness, and response activities; however, HHS should make the results of those investigations and the resources developed therein available to members of the research community and other invested organizations. For example, research into cystic fibrosis, a rare disease that makes individuals particularly susceptible to acute and chronic infections, would disproportionately benefit from innovative approaches and technologies for the detection, characterization, and sequencing of pathogens (and other topics related to pathogen genomics) that may result from this legislation.

**Sec. 232. Vaccine distribution plans.**

The revision to the Public Health Service Act to encompass non-influenza pandemics is a critical step to addressing future public health crises. However, future vaccine distribution plans must also consider the needs of high-risk individuals, including the location and accessibility of vaccination sites, additional protection for vulnerable populations, and criteria by which high-risk and underserved populations are prioritized. We wish to specifically point out that rare diseases require distinct consideration in the prioritization process. It can be difficult to gather enough evidence to accurately characterize disease risk and severity without large patient populations. This may result in an underestimation of the true impact of diseases such as COVID-19 on the individuals with rare diseases like CF, and lack of recognition of the true risk for this population. We therefore urge the committee to ensure that vaccine distribution

plans account for the unique challenges with rare disease populations and provide special consideration for these patients.

#### **Sec. 501. Advancing qualified infectious disease product innovation.**

We applaud the committee for recognizing the severe and growing threat of infectious diseases—as well as the promise of biological products as tools for countering them, as indicated by the expansion of the qualified infectious disease product designation to include biological products. These products, including bacteriophage therapies, hold great promise for the treatment of drug-resistant infection in CF.

COVID-19 has also highlighted the ongoing problem of antimicrobial resistance (AMR) and this legislation should take steps to address this issue by including the bipartisan PASTEUR (Pioneering Antimicrobial Subscriptions to End Up surging Resistance) Act. As it stands, factors unique to antibiotics—namely, short duration of use and the need to use them judiciously to prevent the development of AMR—make it extremely difficult for companies to earn a return on investments in antibiotic innovation. The PASTEUR Act would first create a subscription model for novel antibiotics through which the federal government may enter contracts with novel antibiotic developers and pay a set fee for a supply of novel antibiotics regardless of the quantity of antibiotics used. This approach pays for value over volume and provides antibiotic developers with the predictable return on investment needed to fuel innovation. The PASTEUR Act would further establish a grant program to support antibiotic stewardship programs in hospitals, with priority given to rural, critical access, and safety-net hospitals. These programs are highly effective at optimizing antibiotic use, reducing resistance, and improving patient outcomes, yet they are typically under-resourced. Given the challenges in the antibiotic market and need to address this issue for future pandemics, the committee should include the PASTEUR Act in this legislation.

#### **Sec. 502. Modernizing clinical trials.**

We appreciate that the committee recognizes the potential benefits of novel approaches to clinical trials, including using digital health technologies, decentralized design, and phase consolidation. The issuance of draft guidances on each of these topics will help guide our efforts to improve clinical trial recruitment, ease the burden of participation for clinical trials, and expedite the drug development process. In the case of seamless, concurrent, and innovative trial designs, we encourage Congress to direct the FDA to issue separate guidances for 1) drugs and 2) biologics such as gene and cellular therapies. This is based on the FDA previously acknowledging that design of early-phase clinical trials of cellular and gene therapy products often differs from design of clinical trials for other types of pharmaceutical products.

#### **Sec. 505. Facilitating the use of real world evidence.**

The limited size of the CF patient population and difficulty of withdrawing CF patients from active treatment regimens have made it increasingly difficult to design and execute traditional randomized controlled trials to develop CF therapies. Given this, we are pleased to see that the PREVENT Pandemics Act acknowledges the necessity of adopting novel approaches to the FDA drug approval process, including the use of real world evidence. We also appreciate that the PREVENT Pandemics Act reflects the value of using real world evidence to support primary approval of new drugs, as well to support the approval of biological products and devices.

## Medication access

People with cystic fibrosis must take increased caution in daily life during a pandemic, which can make it challenging to get necessary medication and medical supplies. It is critical therefore that the committee direct healthcare plans over which it has jurisdiction to allow early refills and medication synchronization, extend refill periods, and provide home delivery of both medications and medical supplies during public health crises. This builds upon current CDC and FEMA (Federal Emergency Management Agency) guidelines recommending that patients keep extra supplies of medications and medical equipment as part of everyday preparedness.

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Once again, we thank the committee for its request for comments on the PREVENT Pandemics Act discussion draft. We look forward to continuing to work alongside the committee in the future on this legislation.

Sincerely,

A handwritten signature in black ink, appearing to read "Mary B. Dwight". The signature is fluid and cursive, with a large initial "M" and "D".

Mary B. Dwight  
Chief Policy and Advocacy Officer  
Senior Vice President of Policy and Advocacy  
Cystic Fibrosis Foundation