



December 20, 2021

The Honorable Diana DeGette
U.S. House of Representatives
2111 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Fred Upton
U.S. House of Representatives
2183 Rayburn House Office Building
Washington, D.C. 20515

Dear Representatives DeGette and Upton:

The Cystic Fibrosis Foundation appreciates the opportunity to provide the following comments on the draft of the 21st Century Cures 2.0 legislation (Cures 2.0). We are pleased that Congress continues to prioritize investments in research with the ultimate goal of accelerating development of innovative treatments. We sincerely appreciate the inclusion of the *Pioneering Antimicrobial Subscriptions to End Up surging Resistance (PASTEUR) Act* and the *Telehealth Modernization Act*, two pieces of legislation we have strongly supported as standalone bills since their respective introductions. Please see below for our full comments on the proposed Cures 2.0 text.

Title I: Public Health

Section 103: Pandemic Preparedness Rare Disease Support Program

We appreciate the acknowledgement that patients with rare diseases can face additional challenges during a pandemic. Throughout the COVID-19 public health emergency, CFF has engaged extensively with state and federal government officials on a variety of issues that impact individuals with CF, including accessing necessary equipment and understanding new telehealth options. Furthermore, the CF Foundation held webinars and established a webpage to help our patients and caregivers navigate the challenges associated with the pandemic.¹

While non-profit organizations like CFF are an important resource for patients, we offer caution about establishing separate rare disease and patient support programs to support access to care during public health emergencies. Instead, we urge Congress to enact policies that provide adequate, affordable health insurance for all Americans to ensure continuous access to care during pandemics and normal circumstances.

Section 105: Developing Antimicrobial Innovations

¹ <https://www.cff.org/managing-cf/covid-19-community-questions-and-answers>

Thank you for acknowledging the urgent need to address the failing antibiotics market and help protect against drug-resistant bacterial infections by including the *PASTEUR Act* in Cures 2.0. CFF has championed this legislation since its initial introduction in 2020 and continues to play a leading role in advocating for its passage. As you know, antibiotics are particularly important for patients with CF, who often struggle with difficult-to-treat infections due to the thick, sticky mucus in the lungs characteristic of the disease.

Modernizing payment and creating new market incentives for these essential medical products is needed now more than ever to ensure novel antibiotic development continues. The subscription model found in the *PASTEUR Act* would provide consistent payments over a period of time to drug developers for access to their product, de-linking revenue from sales volume. Innovative subscription contracts have been used to secure access to other treatments important to public health efforts, such as in the case of Louisiana entering into a subscription contract to pay for hepatitis C treatments.² Additionally, some countries such as the United Kingdom are already exploring how these innovative payment models may work for antibiotic products at a national level.³ The *PASTUER Act* would also incentivize the development of antibiotic and diagnostic stewardship guidelines to encourage appropriate use of antibiotics and includes critical transition measures to stabilize the fragile antibiotic ecosystem in the near-term.

Title II: Patients and Caregivers

Section 203: Increasing Diversity in Clinical Trials

Not unlike other individuals with serious and chronic conditions, people living with CF may face onerous barriers to clinical trial participation. In particular, patients with limited income face substantial barriers to participation. Financial toxicity associated with caring for a chronic condition can be prohibitive. Delays in reimbursement for travel can severely impact a person's finances and ability to continue participation in the trial. This is especially true for rare disease trial participants, who often must travel a significant distance to reach a clinical trial site. Additionally, the amount of time a patient must take off from work, school, child or elder care, or other commitments for clinical trial activities can also be a major deterrent to participation. Taking time off of work or other responsibilities to participate in trials can be challenging for individuals with chronic conditions who may need to reserve time off for routine care and unexpected illness.

Furthermore, CF affects many people of different racial and ethnic backgrounds. However, for many years there has not been adequate representation of people of color shared in the stories and descriptions of the disease by medical and public health entities, as well as by the CF Foundation and the CF community. Improving the representation of people of color within the CF community and addressing health disparities that exist within these groups is critical for the advancement of health equity and the

² <https://ldh.la.gov/news/5181>

³ <https://www.gov.uk/government/news/development-of-new-antibiotics-encouraged-with-new-pharmaceutical-payment-system>

Foundation's mission of serving all people with CF. We have engaged^{4,5} with both the FDA and NIH as both agencies are actively seeking ways to increase diversity in clinical trials.

While we share Congress' interest in increasing diversity in clinical trials, we do not believe any further legislative efforts are needed at this time.

Section 204: Patient Experience Data

The CF Foundation recognizes the need to learn more about patient experience data and the role it could play in clinical trial design; however, we have concerns requiring sponsors to collect this data before we know how it correlates with patient outcomes. Moreover, the FDA is already working to better understand how to incorporate patient experience data in drug development and regulatory decision making through its Patient-Focused Drug Development Guidance Series for the Incorporation of the Patient's Voice in Medical Product Development and Regulatory Decision Making.⁶ This is an ongoing initiative that seeks to address how stakeholders can collect and submit patient experience data and other relevant information from patients and caregivers for medical product development and regulatory decision making. Therefore, CFF does not believe further legislative efforts are needed at this time.

Title III: Food and Drug Administration

Section 302: Grants for Novel Trial Design and Other Innovations in Drug Development

Thank you for highlighting the need for additional resources to advance science around innovative clinical trial designs and further utilization of patient-focused drug development in the Cures 2.0 draft legislation. Rare disease communities often face unique challenges such as small patient populations, poor disease characterization, and a wide range of disease presentation that make it more difficult to carry out traditional clinical trials. FDA innovations in regulatory science, modernization of clinical trial design, and increasing emphasis on the importance of patient input have all been critical in addressing the many challenges rare disease drug development programs face. Further resources will help the FDA to build upon the progress the agency has made to date in these areas.

Section 303: FDA Cell and Gene Therapy

While we appreciate Congress' interest in cell and gene therapy and the need to reduce regulatory barriers for the FDA, the CF Foundation believes there are systems already in place for Congress to receive this information from the FDA in a less onerous way than a new formal report.

However, there are ways Congress could intervene beyond the proposed text to make cell and gene therapies more accessible. Cell and gene therapies hold tremendous potential as treatments for genetic diseases like cystic fibrosis. The CF community is preparing for the potential transformative impact that

⁴ <https://www.cff.org/sites/default/files/2021-10/CF-Foundation-Comments-on-FDA-Draft-Guidance-On-Enhancing-Clinical-Trial-Diversity.pdf>

⁵ <https://www.cff.org/sites/default/files/2021-10/CF-Foundation-Response-to-NIH-RFI-on-Data-and-Biospecimen-Sharing.pdf>

⁶ <https://www.fda.gov/drugs/development-approval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporation-patients-voice-medical>

genetic-based therapies will have for people living with the disease. Effective genetic-based therapies may reduce the overall disease burden and cost to the health care system in the long term. We know, however, that these complex therapies come with a substantial price tag, which complicate coverage under traditional payment models. Thus, it is important that we explore innovative payment methods, such as value-based payments and subscription models, to address these challenges. Pilot programs are needed to find new payment methods that both appropriately reward for the value of the product to a patient and reduce the burden of one-time large sum payments per treatment to the overall system.

As policymakers advance payment system reforms that reward innovation while still ensuring access to innovative therapies, we encourage Congress to consider integrating patient preferences into new payment mechanisms for curative treatments, designing insurance benefits to incentivize highly effective therapies, and focusing on the need to ensure patient access to curative therapies regardless of who they are or where they live. As payers test outcomes-based contracts, where payments are structured over time and are tied to demonstrated benefits of the drug, knowing what is meaningful to patients is paramount.

Section 304: Increasing Use of Real-World Evidence

For people with CF and other rare disease, Real World Evidence (RWE) holds promise in addressing inherent challenges in rare disease drug development. However, not all real-world data sources will be adequate for addressing questions related to care and treatment benefit. We must be thoughtful in assessing what best practices and standards are for generating and validating RWE for these purposes. We have been pleased with the progress the FDA has made to date on RWE and are engaged with the agency as it continues to evolve its thinking on RWE in regulatory decision-making. At this time, we do not believe any further legislative efforts are needed to advance regulatory applications of RWE.

Section 306: Establishment of Additional Intercenter Institutes at the Food and Drug Administration

The CF Foundation opposes the establishment of an intercenter institute for rare diseases as it would create a burdensome requirement to establish an additional structure within the FDA. Rare diseases are heterogeneous and affect every system of the body, which renders them not easily centralized in a center of excellence.

The Foundation has made significant investments in research, drug development, and highly specialized multidisciplinary care that have dramatically changed what it means to live with CF. The FDA plays a critical role in developing and bringing to market breakthrough technologies and therapies that treat the underlying cause of CF or complications caused by the disease. Some of these therapies were reviewed in record-breaking time and have been pivotal for improving outcomes for people with CF. For rare disease products, having the appropriate expertise is necessary for understanding the nuance of the disease as well as the unique challenges experienced by the patient community. Advancements over the last decade would not have been possible without the in-house expertise the agency has already cultivated. CFF believes the establishment of a rare disease center of excellence could hinder the advancements necessary to people with CF and detract from the work that is already being done by the FDA.

Title IV: Centers for Medicare and Medicaid Services

Section 403: Extending Medicare Telehealth Flexibilities

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CFF applauds the inclusion of the *Telehealth Modernization Act* in Cures 2.0 in order to extend and expand access to critical telehealth services after the public health emergency (PHE). Through the COVID-19 response bills, Congress has provided important waiver authority to the Department of Health and Human Services to bypass statutory restrictions on Medicare coverage of telehealth services, and telehealth usage during the pandemic demonstrates the need to extend and expand these flexibilities beyond the PHE.

Prior to the COVID-19 pandemic, Medicare rules largely limited use of a patient's home as the originating site to those living in rural areas or with a specific condition. The drastic increase in telehealth usage during the PHE has demonstrated the burden of geographic restrictions and shown it is appropriate and safe for all patients to receive care from their homes. This legislation permanently removes the antiquated location requirements in Medicare, permitting people with CF to access their established care team from their homes.

Beyond eliminating originating site restrictions, the COVID-19 PHE waivers have increased the types of providers who are eligible to provide telehealth services. The CF clinical care team includes physicians, nurses, dietitians, social workers, and respiratory therapists – each of whom plays a unique role in managing CF care. Having access to all members of the care team helps patients better maintain and manage their care, leading to more consistent and better outcomes. This legislation enables the HHS Secretary to maintain and expand providers eligible to deliver telehealth services, therefore increasing access to care and improving care continuity for patients with CF.

Section 407: Expanding Access to Genetic Testing

The CF Foundation supports efforts to expand access to genetic testing, including next-generation sequencing and genomic sequencing. Genetic testing and sequencing is a crucial tool for people with CF who are not diagnosed through newborn screening (who are disproportionately people of color). Particularly with available genetically-targeted therapies, it is imperative that people with CF know their genotype. However, the cost of such testing continues to be a barrier to access. We support Congress creating a pathway for states to increase frequency of covered genetic testing diagnostic services under Medicaid and to reduce barriers to genetic testing beyond just the Medicaid population. We encourage Congress to explore strategies to increase coverage for genetic tests as clinically appropriate, in all public health programs and private health insurance arrangements.

Conclusion

The CF Foundation thanks Representatives DeGette and Upton for the opportunity to provide comments on the Cures 2.0 draft legislation. We were delighted to see the inclusion of the *PASTEUR Act* and the *Telehealth Modernization Act* and hope our comments on other sections are constructive. We stand ready and willing to be a cooperative partner in this effort.

Sincerely,

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