CYSTIC FIBROSIS FOUNDATION®

July 17, 2020

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:

On behalf of the Cystic Fibrosis Foundation, I write in response to the recent request for input on the proposed Cures 2.0 legislative effort. We appreciate this further opportunity to comment on the concept paper and share priorities for the cystic fibrosis community.

Background on Cystic Fibrosis and the CF Foundation

The CF Foundation is a national organization dedicated to providing all people with cystic fibrosis the opportunity to lead long, fulfilling lives, including supporting research and development for new CF therapies. Cystic fibrosis is a rare, life-threatening genetic disease that affects more than 30,000 people in the United States. When the CF Foundation was formed in 1955, no CF-specific drugs existed. However, by raising and directing funds needed to fuel drug development programs, the CF Foundation has encouraged pharmaceutical companies to invest in research for this rare disease. Today, there are 14 therapeutic products available in the United States to treat people with cystic fibrosis, four of which treat the underlying cause of the disease.

With the recent U.S. Food and Drug Administration (FDA) approval of Trikafta®, we anticipate that approximately 90 percent of the CF community will eventually benefit from available disease-modifying therapies. However, we recognize that not all people with CF will benefit from this new therapy or other disease-modifying treatments already on the market. To accelerate progress towards a cure for all people with cystic fibrosis, the CF Foundation launched our Path to a Cure research initiative. We have committed $500 million over the next five years to support this ambitious research agenda, which aims to further development of new treatment methods for the underlying cause of the disease such as gene replacement and gene editing. As part of this effort, the Foundation recently invested $14 million to develop a vehicle to deliver a healthy version of the defective gene that causes cystic fibrosis.

Despite immense progress in recent decades, there is still critical work to be done to ensure all those living with the disease have access to effective therapies and, ultimately, a cure. From our perspective, the most important and challenging work in CF is still ahead.

Considerations for Coverage and Reimbursement Reforms in Cures 2.0

General Coverage Modernization

Federally financed health insurance programs provide coverage for more than half of people living with cystic fibrosis in the US. Approximately ten percent of people with CF are covered by Medicare, including one in four adults over age 26. Medicaid also provides primary or secondary coverage to 55 percent of children with CF

and about a third of adults.[1] It is critical that these programs provide timely access to the care and treatment beneficiaries need, as delays or barriers to care can severely impact health and quality of life for patients with CF and others with chronic conditions. As you move forward with Cures 2.0 legislation, we urge you to focus on improving timely coverage for newly approved therapies, ensuring affordability for patients, and incorporating input from rare disease experts throughout the drug approval and coverage process.

Medicaid

Medicaid is a critical source of coverage for people with cystic fibrosis, and the requirement that Medicaid cover all medically necessary drugs remains a vital protection for people with cystic fibrosis. While the CF Foundation appreciates the reality that growth in drug costs contributes to the increasing budget strain, introducing new coverage restrictions could pose dangerous barriers to care for people with CF. Available treatments for cystic fibrosis are few and not interchangeable; more than one drug per class is necessary in some therapeutic areas such as cystic fibrosis transmembrane conductance regulator (CFTR) modulators used to treat the underlying cause of the disease, inhaled antibiotics, and pancreatic enzymes to meet the diverse needs of those living with CF. Moreover, as highly effective treatments—and ultimately a cure—emerge from the CF drug pipeline, coverage and reimbursement for these therapies should be swift and equitable. The FDA review and approval process is the world's gold standard for evaluating safety and efficacy. State Medicaid programs are not equipped to perform additional clinical effectiveness evaluations and should reference the FDA label indications when making coverage decisions. We encourage Congress to work with Centers for Medicare and Medicaid Services (CMS) to ensure state Medicaid programs use FDA labels as the basis for any coverage decision and adhere to the requirement to cover all medically necessary drugs.

In addition to current coverage standards, Medicaid programs should provide publicly available timelines for reviewing newly approved products, and CMS should establish a national timeline by which coverage reviews must take place. Too often states delay reviews for months or even more than a year, which in turn can delay access for individuals and cause further deterioration in patients' health. While many states offer case-by-case reviews in the interim, this is not sufficient to guarantee coverage for those who need it. Case-by-case reviews are typically conducted without standard coverage criteria, leading to subjective approvals and denials depending on who reviews each request.

Finally, state Medicaid programs should be required to provide opportunities for public input from patients and clinical experts during drug reviews (typically conducted by Pharmacy and Therapeutics Committees and/or Drug Utilization Review Boards), especially for rare diseases for which committee members likely lack an understanding of treatment and patient experience. These processes for providing public input should be transparent, including publicly posted agendas at least a month in advance of a meeting and opportunity for both written and verbal input from patients and clinicians.

Medicare

The majority of people with CF with Medicare coverage qualify through disability and are living with more severe disease. The most significant challenge Medicare beneficiaries living with cystic fibrosis face is affordability. Unlike other forms of coverage, Medicare does not limit out-of-pocket spending except in certain Medicare Advantage plans. This is especially problematic for beneficiaries with Part D plans who rely on high-cost drugs. For people with CF—who are often on multiple specialty therapies, including a CFTR modulator—the costs incurred can be extremely high, even after reaching a "catastrophic" level of out-of-pocket spending. For most individuals with CF, paying five percent co-insurance in the catastrophic phase is impossible and can lead to discontinuation of lifesaving, albeit expensive, medications for the remainder of the plan year. It is

[1] https://www.cff.org/Research/Researcher-Resources/Patient-Registry/2018-Patient-Registry-Annual-Data-Report.pdf

essential that Congress pass an out-of-pocket cap for Medicare Part D to make current therapies and future cures affordable to those who need them.

Cell and Gene Therapies

Congress should consider establishing pilot programs to help advance alternative payment models for cell and gene therapies. Like many other rare disease communities, the CF community is preparing for the potential transformative impact that 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. Tools such as patient-reported outcomes (PROs) should be used to ensure patient voice is considered in payment decisions. We believe that any policy solutions to pay for cures should enable affordable access for all eligible patients.

Medical Products for Small Patient Populations

Thank you for acknowledging the importance of increasing diversity in clinical trials by reducing barriers to participation. For many small rare disease communities, finding and engaging enough patients in clinical trials can be a particularly difficult barrier to overcome and can dampen efforts to develop much-needed therapies. Therefore, with small patient populations, it is especially critical that we ensure patients have access to clinical trials in order to make progress on developing new treatments. We encourage Congress to consider further solutions for reducing barriers to trial participation and increasing the overall diversity of clinical trial participants.

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.

While ensuring Medicaid covers routine care costs associated with trial participation is an important step, more can be done to address financial barriers. Drug sponsors should be encouraged to use reimbursement practices that work for patients at all income levels, such as reimbursing patients on the day of a visit or covering the costs of flights and hotels upfront. Additionally, drug sponsors need a clear signal that reimbursement for childcare and eldercare costs is acceptable and not considered coercive. We strongly

encourage Congress to work in concert with the FDA to improve practices around reimbursing clinical trial participants.

Congress should also consider alternative methods to ensure adequate reimbursement is available for low-income individuals. One program that may serve as a model for reimbursing clinical trial participants is the Health Resources and Services Administration (HRSA) reimbursement program for living organ donors.[2] This program addresses financial disincentives by providing vital support for individuals who are interested in becoming a living donor but who would otherwise lack the financial means to cover associated expenses. HRSA's program is meant to be a payer of last resort in the circumstance that other payers are not available to make up for costs associated with being a living donor. Creating a program similar to the HRSA reimbursement program for living organ donors may provide a practical solution for ensuring all individuals who wish to participate in clinical trials and are eligible can do so.

Finally, we ask you to consider further ways that Congress, in concert with the FDA and CMS, can ensure clinical trials work better for patients, including exploring ways to integrate telehealth into trial designs. The COVID-19 pandemic has made the value of telehealth more apparent than ever before. Access to telehealth services during the coronavirus pandemic has been especially important for those living with CF, who may be at increased risk for developing serious illness related to COVID-19. Importantly, telehealth and other remote services can also enable less burdensome clinical trial participation. Increasing access for trial participants to remote monitoring devices, such as at-home spirometry to measure lung function, or flexibility in how visits are conducted and samples are collected by using local lab facilities rather than the study site for collection of blood samples can greatly reduce the burden of trial participation. Trial designs that make clinical trials more accessible to participants, such as remote or mixed remote and in-person studies, should be encouraged where feasible.

Breakthrough Coverage

We've seen that the FDA is more than capable of completing swift reviews and approvals of new products with current authorities the agency has at hand when the evidence generated from clinical trials is clear. All four CFTR modulators used to treat the underlying cause of cystic fibrosis have received Breakthrough Therapy designation. Most recently, the FDA leveraged Priority Review, Breakthrough Therapy, and Orphan Drug designation during the approval process for the triple combination treatment of elexacaftor/tezacaftor/ivacaftor (Trikafta™), a drug that treats the underlying cause of CF for up to 90 percent of the population.

This review is a model of success for incorporating rare disease experts into the review process without compromising a thorough evaluation of drug safety and efficacy. We advise Congress against prioritizing speed at the expense of ensuring products approved are safe and effective. To better serve the patients, Congress should instead ensure that the FDA is adequately staffed and resourced so rare disease drug sponsors can engage early and often with the agency to ensure study designs result in meaningful and reliable results.

Additional Feedback on the Cures 2.0 Concept Paper Policy Proposals

COVID-19 Rare Disease Support Program and Pandemic Preparedness Program for Patients

While we appreciate the acknowledgment that patients with rare diseases can face additional challenges during a pandemic, 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

[2] https://www.livingdonorassistance.org/About-Us/Mission-Background

provide adequate, affordable health insurance for *all* Americans to ensure continuous access to care during pandemics and in normal circumstances.

Congressional and administrative action in response to COVID-19 has underscored the need for comprehensive, affordable coverage. For instance, Congressional relief bills provided that diagnostic testing be available with no-cost sharing under Medicaid, Medicare, TRICARE, Veterans Affairs, and other coverage and created an option for Medicaid to cover diagnostic testing for the uninsured at 100 percent federal match. The administration used its authority to reimburse providers treating uninsured patients with COVID-19 as well. While these were necessary actions, this piecemeal approach does not ensure all Americans have access to COVID-19 testing and treatment. Only 21 states have adopted the option to provide diagnostic testing though Medicaid.[3] The requirements also do not apply for those enrolled in short-term limited-duration plans and other non-ACA-compliant coverage. Finally, it is not clear whether the funds used to reimburse providers treating uninsured patients with COVID-19 will be sufficient to cover their costs. Taken together, these actions highlight the need for holistic policy solutions to ensure all American—those with rare diseases and otherwise—can access needed care during pandemics to minimize the risk to themselves and others.

Improving U.S. Pandemic Preparedness and Response through Support of Antimicrobial Resistance Product Commercialization

Thank you for acknowledging the urgent need to address the failing antibiotics market to help protect against drug-resistant bacterial infections. 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. We urge Congress to establish new postmarket antibiotic development incentives to ensure we have the tools to fight off dangerous resistant infections both now and in the future.

Modernization in payment and new market incentives for these essential medical products are needed now more than ever to ensure novel antibiotic development continues. Payment methods that de-link sales volume from revenue can help. One such model, sometimes referred to as the 'Netflix' subscription model, would provide consistent payments over a period of time to drug developers for access to their product. 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.[4] 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.[5]

Another reimbursement fix, put forward in the Developing an Innovative Strategy for Antimicrobial Resistant Microorganisms (DISARM) Act (S. 1712, H.R. 4100), would carve out antibiotics from Medicare inpatient reimbursement and provide a separate additional payment for novel antibiotics. This measure would provide some immediate relief to antibiotic companies that are struggling to stay in business. Furthermore, innovative incentives like a market entry reward for novel antibiotics may increase interest in this development space. We urge you to consider including these and other solutions in Cures 2.0 to ensure access to antibiotics that work both now and in the future.

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[3] https://www.kff.org/coronavirus-covid-19/issue-brief/medicaid-emergency-authority-tracker-approved-state-actions-to-address-covid-19/; https://www.kff.org/coronavirus-covid-19/issue-brief/eligibility-for-aca-health-coverage-following-job-loss/

[4] http://ldh.la.gov/index.cfm/newsroom/detail/5181

[5] https://www.gov.uk/government/news/development-of-new-antibiotics-encouraged-with-new-pharmaceutical-payment-system

FDA Grant-making Authority and Funding

Thank you for highlighting the need for additional resources to advance the science around innovative clinical trial designs and further utilization of patient-focused drug development in the Cures 2.0 concept paper. 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 designs, 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 on the progress the agency has made to date in these areas.

Increasing Use of Real-World Data/Evidence

For people with CF and other rare diseases, 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.

Instead, we encourage Congress to explore applications for RWE in optimizing care and supporting payment decisions. The data generated from electronic health records (EHRs), wearables, and other digital health technologies may reveal valuable insights on the health of our patients and the efficacy of existing treatment interventions. There is a considerable amount of work that still needs to be done to ensure these tools can generate meaningful data that is fit for use as evidence for care and payment decisions.

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Once again, we thank you for this opportunity to provide input on Cures 2.0. There are important opportunities for collaboration and discussion on reforms to improve patient access to innovative and curative treatments. We stand ready to work with Congress on the challenges ahead. Thank you for your consideration.

Sincerely,

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