



December 16, 2019

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 request for input on the proposed Cures 2.0 legislative effort. We appreciate this opportunity to comment on the focus areas you have identified for Cures 2.0 as well as priorities for the cystic fibrosis community.

We urge that your focus on this legislation be first and foremost on patients. We are pleased that Congress continues to prioritize investments in research with the ultimate goal of accelerating innovative treatments for the benefit of patients. Any treatments developed as a result of public investments must be both meaningful and accessible for patients to benefit. We hope that you will not lose sight of that goal as you proceed in crafting this new piece of legislation.

Our comments below reflect our hope that Cures 2.0 will strive to reflect the experiences and needs of today's patients while also anticipating the changing therapeutic environment that will impact the care of tomorrow's patients.

Background on Cystic Fibrosis and the CF Foundation

The CF 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. When the CF Foundation was formed in 1955, no CF-specific drugs existed. However, by raising and directing funds needed to fuel CF drug development programs, the CF Foundation has encouraged pharmaceutical companies to invest in rare disease research. Additionally, with the CF Foundation's Therapeutics Development Network (TDN) – the largest CF clinical trials network in the world – we have been able to spur clinical trial designs that work for the cystic fibrosis community. Today, there are 14 therapeutic products available in the United States to treat people with CF, four of which treat the underlying cause of the disease.

With the recent 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 recently 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. From our perspective, the most important and challenging work in CF is still ahead.

We are dedicated to providing all people with cystic fibrosis the opportunity to lead full, productive lives, and we recognize that 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.

How Congress Can Help

The CF Foundation is doing our part to further therapeutic development and standards of care for people living with CF, but we cannot do it alone. The federal government continues to be a key partner in our efforts to improve the lives of people with cystic fibrosis. Cures 2.0 offers an opportunity to pursue much-needed health care system reforms to improve patient access to innovative and curative treatments. We believe that Congress should focus on reforms that will ultimately foster a modern and sustainable health ecosystem that prioritizes patient benefit in an era of curative therapies.

In particular, we believe that a 21st century patient-focused health ecosystem will need different methods for:

- Envisioning a drug approval process for especially small pools of patients in an era of highly personalized medicine;
- Utilizing the data we have available to tackle new and existing health challenges; and
- Paying for innovative therapies and cures as well as medicines of high societal value.

If approached thoughtfully, we believe that Cures 2.0 has the power to tackle some of the most onerous barriers to patient-centric care as we approach this new era of curative therapies. We ask that you keep these issues in mind as you consider next steps for a Cures 2.0 legislative package.

Considerations for Priority Areas for Reform in Cures 2.0

Real World Evidence has the power to support care optimization and payment decisions.

For people with CF and other rare diseases, Real World Evidence (RWE) holds promise in addressing inherent challenges in rare disease drug development. We believe it is important to move forward in a thoughtful manner in applying RWE in regulatory decision-making. RWE has limitations that must be acknowledged, and the capabilities of this data must be further assessed to ensure study outcomes are valid and reliable. We have been pleased with the progress the FDA has made on RWE and are engaged in the process as the Agency continues to evolve its thinking on RWE in regulatory decision-making.

We do not believe any further legislative efforts are needed at this time 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. However, not all real-world data sources will be adequate for addressing questions related to care and treatment benefit. 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. We must be thoughtful in assessing what best practices and standards are for generating and validating RWE for these purposes.

Digital health technology utilization can be advanced through payment reform.

We appreciate your call for thoughtful improvements to how digital health technologies are utilized in the health care system. Patients in the CF community have long benefited from intentional data collection and utilization, which has helped to spur important changes in standards of care and care delivery for people living with cystic fibrosis. As mentioned above, digital health technologies are already key sources of real-world data. Advances in how digital health care is delivered and covered may yield further benefits for small patient communities like CF by reducing the need to travel for specialized treatment and increasing opportunities for data on health status to be shared with providers to assist with care decisions.

Improved payment practices for telehealth as well as insurance coverage for wearable technologies can help to increase utilization of available digital health tools. Making telehealth and other digital technologies more accessible through payment reforms may help reduce barriers to care access and improve quality of care in some instances. However, it is important to note that not all care can be appropriately delivered through digital technologies. Meaningful changes in accepted utilization and payments for digital health technologies will need to go hand-in-hand with efforts to improve access to more traditional forms of care for patients to truly benefit.

While we are hopeful for future advances in health delivery through digital technologies, there are existing challenges to patient access to care that can be meaningfully addressed in Cures 2.0. Medical record access is critical for many patients with complex, chronic conditions like cystic fibrosis, as medical history often dictates what treatments are best for a given patient. The Medical Records Access Fairness Act (H.R. 4698) seeks to strengthen a patient's right to access their medical records by removing cost barriers to patients looking to obtain medical record copies. We ask that Congress consider H.R. 4698 and other efforts to ensure that all patients, including people with CF, can obtain copies of their medical records regardless of their financial circumstance.

Payment methods for both curative therapies and essential products like antibiotics must be modernized.

Curative Therapies: Gene therapy development has been advancing rapidly, and the promise of these new treatments makes this an exciting time in medicine. As Congress looks for payment system reforms for appropriately compensating drug developers while ensuring access to innovative therapies, we ask that you 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 patients can access curative therapies regardless of who they are or where they live.

As the system moves toward 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. Additionally, high deductible health plans or other plan designs that require high cost sharing from enrollees could make a cure unaffordable for many patients. We believe that any policy solutions to pay for cures should enable affordable access for all eligible patients.

Antibiotics: Beyond curative therapies, Congress can and should act now to address the failing antibiotics market through reimbursement reforms. While some new antibiotics are in development, almost all are derived from antibiotic classes discovered decades ago. Only a handful of new antibiotics are developed each

year, leaving much of the antibiotic treatment needs unmet. Many patients – including people with CF, who are particularly prone to difficult-to-treat acute or chronic respiratory infections – rely on the availability of effective antibiotics to protect them from otherwise life-threatening infections. Modernization in payment for these essential medical products is needed now more than ever.

CMS has signaled the importance of reimbursement reform for antibiotics, but more needs to be done to protect the advances of modern medicine that are built off of effective infection treatment options. Reimbursement reforms such as the software licensing model previously proposed by the FDA have the potential to be effective in impacting the number of drugs that make it to market.

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. Importantly, payment reforms that delink sales volume and revenue will help incentivize novel antibiotic development while reducing concerns about inappropriate use of these critical products.

Reimbursement reform alone will not fix the broken antibiotics market. Products in the antibiotics pipeline would ideally be supported by a robust ecosystem of incentives to combat the low market, high societal value of novel antibiotics. Early development phase interventions, while beneficial, have not been enough to stabilize the antibiotics drug development pipeline. Current successful practices and programs that assist sponsors during early product development, such as CARB-X, could be expanded to cover additional sponsors or additional phases of development. Furthermore, innovative incentives like a market entry reward for novel antibiotics may increase interest in this development space. We ask that Congress consider further opportunities to incentivize antibiotic development.

Additional Opportunities to Accelerate Cures for Patients

Access to life-saving cures starts in the lab.

Many of the basic research findings that led to the development of disease-modifying treatments for people living with CF – including the discovery of the CF gene – were the result of strong public research investments. Additional basic questions related to cystic fibrosis are being studied in laboratories across the country by investigators supported at least in part by NIH.

This continuous cycle of research and development is critical to our efforts to improve care for those with CF, and we urge Congress to consider the adverse impacts that uncertainty in the annual federal funding process has on scientific progress. Congress has made a concerted effort to address the falling purchasing power of NIH through the funding provided in the 21st Century Cures Act and the substantial increases in annual funding over the last few years. Creative methods for supporting US biomedical research that mitigate the problems brought on by the annual appropriations process should be considered. Additionally, while it may be early to start thinking about extending authorized funding for 21st Century Cures activities, we urge you to consider creating a stable funding stream for Cures-initiated activities at the NIH.

Federal supports are also vital for cultivating the research workforce of the future. 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. NIH has made progress in making R01 grants more accessible to first-time recipients, but more work is needed to ensure junior investigators can progress in their field. Making the second R01 grant more obtainable will encourage young scientists to continue their work and improve upon NIH's efforts to support young researchers.

The FDA will be a key asset in getting cures into the hands of patients.

A well-staffed and well-resourced FDA is critical to patient groups as well as the drug industry. With science growing more complex, FDA needs to be resourced to prepare the Agency for assessing the new technologies and science that will deliver tomorrow's cures. Ensuring the FDA is able to compete with the private sector to secure the appropriate expertise in-house is vital for ensuring the highest regulatory standards during the medical products review process.

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. The FDA was able to review four CF-specific drugs, some in record time, which would not have been possible without the in-house expertise the Agency has been able to cultivate.

Again, we urge you to begin thinking about how to ensure the funding provided through the 21st Century Cures Act can continue past its expiration.

Coordination on international clinical trials is needed to speed progress.

Improved coordination on international clinical trials will be important for supporting development of new treatments as clinical trials for both adults and children need to draw on global patient populations with increasing frequency. This is especially true for rare disease populations like CF, where trials may need to access populations of patients from around the world in order to overcome small patient pools in any given country. As a result, it is imperative that clinical trial designs, endpoints, and the timing of studies are coordinated between global regulatory agencies such as the European Medicines Agency and the FDA. While FDA is already engaged in these efforts, Congress could make it clear that this is a priority.

Cures can't happen without patient access to clinical trials.

For many people with CF and other serious chronic illnesses, clinical trials represent an important opportunity to access potentially life-changing treatments and prevent further health decline. Trials also present a valued opportunity for individuals to contribute to advances that can benefit the broader disease community. However, there are many barriers that deter patients from taking advantage of opportunities to participate in trials.

Challenges related to small patient population size, language and cultural barriers, and financial burden associated with trial visits should be tackled through more thoughtful and patient-centric clinical trial designs and enrollment practices. An individual's circumstances in life should not prevent them from participating in clinical trials. Not all patients have access to reliable income, transportation, childcare, or the other resources needed to proactively participate in trials. Alleviating barriers such as these can increase the pool of trial participants and help accelerate the drug development process.

The FDA has already taken steps to combat clinical trial access barriers, including a recent draft FDA guidance that offers practical advice to sponsors on how they can improve trial enrollment practices. Congress should

take further steps to empower patients who want to participate but may face particularly onerous barriers to doing so.

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,

A handwritten signature in black ink, appearing to read "Mary B. Dwight". The signature is fluid and cursive, with a long horizontal stroke extending to the right.

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