NATIONAL HEALTH COUNCIL

FDA User Fee Reauthorization: Ensuring Safe and Effective Drugs and Biologics
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United States House Committee on
Energy and Commerce
Randall L. Rutta
Chief Executive Officer

Chairwoman Eshoo, Ranking Member Guthrie, and Members of the Subcommittee on Health of the Committee on Energy and Commerce, on behalf of the National Health Council (NHC), I am writing to share our perspective on the reauthorization of user fee agreements (UFA) for fiscal years (FY) 2023-2027.

Background on the NHC

Created by and for patient organizations 100 years ago, the NHC brings diverse organizations together to forge consensus and drive patient-centered health policy. We promote increased access to affordable, high-value, sustainable health care. Made up of more than 140 national health-related organizations and businesses, the NHC's core membership includes the nation's leading patient organizations. Other members include health-related associations and nonprofit organizations including the provider, research, and family caregiver communities; and businesses representing biopharmaceutical, device, diagnostic, generic drug, and payer organizations.

We thank you for the opportunity to submit our recommendations ahead of the Congressional hearing and look forward to providing further support to continue innovating the regulatory framework on behalf of people with chronic conditions and disabilities across the United States (U.S.).

FY2023-2027 User Fee Reauthorizations

We are pleased to see Congress' efforts toward the reauthorization of the UFAs for FY2023-2027. We support the user fee goals outlined in the Food and Drug Administration's (FDA) Commitment Letters, which support timely reviews of products while ensuring a rigorous approach to determining safety and efficacy as well as demonstrate a focus on the patient perspective. As members of the Energy and Commerce Committee are aware, UFAs are an essential vehicle for ensuring FDA has needed capacity to review products and modernize the regulatory infrastructure, which is a critical mechanism for addressing many of the health care issues patients face today.

The importance of the user fee reauthorizations is particularly timely this upcoming cycle given the inequitable access to COVID-19 vaccines and treatments, weaknesses in the drug supply chain, and need for increased focus on comorbidities and minority populations in clinical trials. At the same time, health care stakeholders have seized opportunities for increasing the efficiency and transparency of the drug review process and modernizing clinical trial protocols, particularly through advanced technologies – processes that need to be firmly incorporated in the overall regulatory framework.

We hope that Congress will partner with the NHC to achieve our goals to further the science of patient engagement; elevate the voice of patients; and focus on more equitable research, development, and delivery of medical products. We are ready to provide any support necessary for the passage of the agreements. The NHC highlights some of the most important initiatives included in the FDA Commitment Letters for the next cycle:

Prescription Drug User Fee Act (PDUFA) VII[1]

- **Patient Focused Drug Development (PFDD) Initiative:** Continued funding and development of the PFDD initiative will be key to ensure that the patient perspective is systematically incorporated in the drug development process. As such, we are pleased to see the inclusion of specific pathways for patient input and *urge Congress to ensure that this initiative remains a key focus of the next user fee cycle.*
- **Digital Health Technologies (DHTs)**: Technology for data collection, storage, and transmission has advanced to harness a strong potential to provide a better picture of patient experience during a clinical trial with timelier and more direct information collection. If deployed appropriately, the technology can also benefit patients more directly by providing them with updates on the clinical trial process and transparency around the use of their data. Wearables and other remote monitoring devices can support drug developers and the FDA by reducing the significant burden of clinical trial participation, enhancing sponsors' ability to collect critical information from a broader, more diverse set of patients during clinical trials. While this area was a priority of the NHC before the COVID-19 pandemic, it has been even further elevated, as many sponsors had to modify their trial protocols to limit the frequency of trial site visits to protect participants from the virus, which potentially offers a treasure trove of lessons. The NHC applauds the FDA's commitment to developing a framework to evaluate the use of DHTs in clinical trials. *Specifically, we are pleased to see that FDA is committed to:*
    - Conducting demonstration projects, including engagement with stakeholders such as patients and patient organizations;
    - Convening a series of public meetings or workshops;
    - Establishing a cross-center committee with the role, among other priorities, of engaging stakeholders such as patient representatives; and
    - Creating a DHT framework document.
- **Advancing Real-World Evidence (RWE) Program:** Previous user fee programs have initiated the RWE Program, which has been instrumental in paving the way for incorporation of broader patient populations in the evaluation of drug products and has received particular attention in the development of COVID-19 vaccines and therapeutics. The PDUFA VII commitments are designed to further the science of RWE. The Program will provide drug developers and the FDA with flexibility for data collection methods that will go a far way to helping those suffering from chronic diseases gain access to critical medicines. RWE can also give us a more robust perspective on benefits of risks medical products on a broader population than limited clinical trial populations. *We strongly support development of the Advancing RWE Program, including FDA funding for new RWE pilots that will support the dissemination of lessons learned on the topic to patients and manufacturers.*
    - We further encourage Congress to consider enhancing the program by including provisions for researchers to engage patients, or leverage insights from patients, in the design, conduct, and translation of real-world

research such that patients' lived experiences are appropriately reflected in drug development.

Biosimilar User Fee Act (BsUFA) III and Generic Drug User Fee Amendments (GDUFA) III

While the BsUFA and GDUFA programs have not existed as long as PDUFA, they are equally critical in increasing affordable access to medicines for patients and have received similar attention as part of larger discussions around drug access in the U.S.

In particular, the availability of generic drugs has delivered tremendous savings to people with chronic conditions and disabilities, and to the health care system more broadly. With generic drugs comprising nine of 10 prescriptions filled in the U.S.[2], the GDUFA program requires significant resources and staff.

We encourage Congress to provide the FDA with the necessary funding and resources to be able to effectively manage the generic drug review process, enhance communication practices between the Agency and sponsors, and advance product-specific guidance, ultimately to increase timely generic availability.

Similarly, the BsUFA program is critical for providing clear and effective regulatory pathways to licensure of biosimilar and interchangeable biologics. These products, as with generics, provide cost-saving opportunities for patients and the U.S. health care system. The biosimilar industry, and its regulatory framework, is faced with unique development and manufacturing challenges compared to small-molecule drugs and generics. As a result, we would like to emphasize the importance for Congress to support initiatives outlined by the FDA focused on enhancing the biosimilar and interchangeable biological product development process and the use of advanced analytical and pharmacological assessments in application submissions.

Historical Importance of User Fee Agreements

The UFAs create a structure for the FDA, industry, and other health care stakeholders to ensure that patients have equitable access to life-saving medicines as rapidly and safely as possible, and that the patient voice is incorporated in the drug development and review process.

The UFAs were initially set up to facilitate access to medicines through a variety of mechanisms: they expedite FDA review of new medicines, devices, biosimilars, and generic drugs; increase resources for the Agency through sponsor and manufacturer fees; and create expectations on behalf of industry and the FDA for the drug development and review process. Past UFAs have been critical in modernizing our regulatory frameworks to focus on patients' needs through various programs and initiatives, such as:

- **PDUFA III** – Reauthorization initiated a "rolling review" pilot program for products addressing rare and life-threatening diseases (called the Orphan Drug Designation), which is an area that still needs significant attention as people continue to suffer without available treatments for rare diseases.
- **PDUFA V** – Under this reauthorization from 2012-2017, the FDA conducted 24 disease-specific Patient-Focused Drug Development (PFDD) meetings to obtain the patient perspective more systematically on specific diseases and their treatments.
    - The agreement also increased funding for the Rare Disease Program which, in later reauthorizations, was expanded by incorporating Program staff into each review division of the Agency.

- **PDUFA VI** – In the last PDUFA reauthorization, we saw the FDA build upon learnings from the PFDD meetings to produce guidance for industry and other stakeholders on collecting systematic patient experience data.
    - FDA also initiated its RWE Program by generating guidance on the use of real-world data (RWD) in the evaluation of safety and efficacy of products and regulatory decision-making.
    - FDA has also advanced guidance around the use of patient reported outcomes in regulatory submissions during this PDUFA period.

Conclusion

The NHC would like to thank the Subcommittee on Health and the entire Committee on Energy and Commerce for their work on the UFA reauthorizations that will help patients in the U.S. gain access to medicines in a timely and safe manner.

As we, hopefully soon, emerge from the COVID-19 pandemic, the agreements will be an imperative vehicle for modernizing our health care system and filling the gaps in care that still exist today. The NHC is ready to work with Congress in any capacity necessary on the issues pertaining to the user fee programs, as well as other topics affecting the health care industry. Thank you for the opportunity to provide the perspective of the NHC and, by extension, millions of people with chronic conditions and disabilities in the U.S.