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October 28, 2021

Janet Woodcock, MD
Acting Commissioner
US Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

RE: PDUFA Reauthorization Performance Goals and
Procedures FY 2023-2027

Dear Acting Commissioner Woodcock:

The National Health Council (NHC) appreciates the opportunity to provide feedback on the Food and Drug Administration's (FDA's) draft performance goals for the Prescription Drug User Fee Act from fiscal years 2023-2027 (PDUFA VII).

Created by and for patient organizations over 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, and payer organizations.

The PDUFA VII draft goals letter demonstrates FDA's commitment to modernizing its regulatory frameworks and activities to meet the demands of 21st Century technologies. Above all, we are pleased to see several continuing or new initiatives implemented with specific requirements to incorporate perspectives of people with chronic diseases and disabilities.

Enhancing the Incorporation of the Patient's Voice in Drug Development and Decision-Making

The NHC applauds the FDA for continuing its crucial work to evolve the science of patient engagement through PDUFA VII. The patient-focused drug development (PFDD) initiative funded by PDUFA V and PDUFA VI demonstrated that both FDA and the pharmaceutical industry recognize the importance of engaging patients and caregivers to gather their perspectives on their condition and desired outcomes of potential treatments. PDUFA VII continues this commitment. Specifically, we are pleased to see:

- Development of a mechanism to engage external experts to support the review of patient experience data;
- Consistent training across review divisions on patient engagement methodology;
- Workshops and a request for information on the submission and evaluation of patient experience data in the context of benefit-risk assessment and product labeling; and
- Additional guidance on use and submission of patient preference information to support regulatory decision making.

Additionally, while not funded through user fees, we are pleased to see FDA's further commitment to developing a catalog of standard core sets of Clinical Outcome Assessments (COAs) and Related Endpoints and the development of a public input process to gather stakeholders' perspectives on diseases and domains of greatest need for a standard core set. The NHC also reiterates the need to broaden this work on core sets to include measures beyond COAs that consider the full range of impacts a disease and its treatments have on patients and families and to ensure the concepts and measures included in core impact sets are developed starting with patient input. We strongly encourage the FDA to expand its work from COA sets to the broader set of core impacts and mechanisms needed to identify those impact sets with patient input. The area of Patient-Centered Core Impact Sets is one where the NHC is very active and welcomes the FDA's engagement.

Enhancing Use of Digital Health Technologies to Support Drug Development and Review

The NHC is incredibly impressed by the FDA's commitment to developing a framework to evaluate the use of digital health technology (DHT) such as wearables and other remote monitoring devices for purposes of collecting information for clinical trials. The NHC has long recognized the potential for these tools to reduce the burden of clinical trial participation and potentially address the lack of diversity in clinical trial populations. This recognition only grew during the COVID-19 pandemic, as trial sponsors had to think creatively about how to reduce the frequency of site visits for participants. However, we also believe additional activities are needed to incorporate the patient perspective into the deployment of digital tools and to provide clarity to sponsors. We are pleased to see the FDA commit to addressing both gaps in PDUFA VII. Specifically, we support:

- Conducting demonstration projects, including engagement with stakeholders such as patient organizations;
- Convening a series of public meetings or workshops with key stakeholders including patients;
- Establishing a cross-center committee with the role, among other priorities, of engaging stakeholders;
- Creating a DHT framework document; and
- Publishing guidance on the use of DHTs in traditional and decentralized clinical trials.

We are very encouraged by the common thread of patient engagement throughout the FDA's proposed efforts on DHTs. The NHC and the broader patient community welcomes the opportunity to work with the FDA to continue to engage patients and the patient community on the DHT framework and all future work on DHTs.

Advancing Real-World Evidence for Use in Regulatory Decision-Making

The NHC is supportive of leveraging real-world evidence (RWE) to inform FDA's decision-making. The proposed provisions for PDUFA VII are a common-sense approach to furthering the science of RWE. We support development of the Advancing RWE Program, which will conduct a series of pilot projects to improve the quality and acceptability of RWE-based approaches in support of new labeling claims, including approval of new indications of approved products or to satisfy post-approval study requirements. We believe if appropriately communicated to future sponsors and the patient community, the lessons learned can inform future uses of RWE and lead to a better and more consistent collection of data and best practices.

One potential enhancement to this program is to encourage researchers to engage patients or leverage insights from patients in the design, conduct, and translation of real-world research that reflects patients' lived experience. We have previously described specific opportunities for patient insights to improve real-world research.¹ We encourage FDA to consider the role of patient engagement when selecting which pilot studies to fund.

Enhancing CBER's Capacity to Support Development, Review and Approval of Cell and Gene Therapy Products

The NHC is pleased to see additional attention paid to regulation of new and promising cell and gene therapy products. These products have already improved the lives of people living with very serious conditions. However, it is still an emerging field, and the

¹ Oehrlein EM, Perfetto EM, Schoch S, et al. Patient-Centered Real-World Evidence: Methods Recommendations from an Evidence-Based Consensus Process. National Health Council, Washington DC, May 2021. Available from: <https://nationalhealthcouncil.org/patient-centeredrwe>

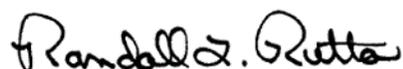
number of applications for cell and gene therapies expected to be submitted to FDA is set to dramatically increase. The reauthorization of PDUFA comes at an ideal time to increase the staff and resources the FDA has to ensure they have the scientific expertise to evaluate the data and make regulatory decisions in a timely fashion.

In addition to the added staff, the NHC is incredibly supportive of the proposed public PFDD meeting to better understand patient perspectives on cell and gene therapies. Patients' views on benefit-risk, desired outcomes, and ability to participate in required post-market studies may vary greatly between treatments with durable effects versus more traditional treatments. This PFDD activity may provide very important learnings for the FDA, sponsors, and the patient community.

Conclusion

The NHC thanks FDA for its commitment to patient engagement as a key feature of its role in delivering safe and effective treatments to people with chronic diseases. We are pleased to see this commitment reflected in the PDUFA VII draft performance goals document and support PDUFA's reauthorization. Please do not hesitate to contact Eric Gascho, Vice President of Policy and Government Affairs if you or your staff would like to discuss these issues in greater detail. He is reachable by phone at 202-973-0545 or via e-mail at egascho@nhcouncil.org.

Sincerely,



Randall L. Rutta
Chief Executive Officer