August 21, 2020

Dockets Management Staff
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Submitted VIA: https://www.regulations.gov

Re: Docket No. FDA-2010-N-0128 – Reauthorization of the Prescription Drug User Fee Act, Public Meeting; Request for Comments

The Cancer Support Community (CSC), an international nonprofit organization that provides support, education, and hope to cancer patients, survivors, and their loved ones, appreciates FDA’s solicitation of stakeholder views on the Reauthorization of the Prescription Drug User Fee Act (PDUFA) for fiscal years 2023 through 2027.

As the largest direct provider of social and emotional support services for people impacted by cancer, and the largest nonprofit employer of psychosocial oncology professionals in the United States, CSC has a unique understanding of the cancer patient experience and our comments will focus on providing the FDA with features that should be added to enhance the effectiveness of the human drug review process through the lens of patients.

Overall, CSC delivers more than $50 million in free, personalized services each year to individuals and families affected by cancer nationwide and internationally. In addition to our direct services, our Research and Training Institute and Cancer Policy Institute are industry leaders in advancing the evidence base and promoting patient-centered public policies to ensure that the patient voice is at the center of the national dialogue.

**Patient Experience Data**

With the passage of the 21st Century Cures law in 2016 and the Food and Drug Administration Reauthorization Act (FDARA) in 2017, Congress heightened the importance of collecting “patient experience data” (PED) that not only includes the physical impacts of a condition, therapy, or clinical investigation/trial but also the psychosocial impacts. Congress’ well-established and strong support for inclusion of PED in clinical trials should empower the FDA to take steps to standardize and formalize the methodology of collecting and using PED.

**FDA Requirement to Sponsors Lacks Clarity and Uniformity**

The FDA currently requires clinical trial sponsors to report what (if any) patient experience data were collected during the trial. To date, there has not been clear and uniform guidance on how
patient experience data could and should be captured and communicated to patients and providers to benefit the shared decision-making process. PDUFA provides the appropriate mechanism and mandate for the FDA to design and implement a mechanism to fulfill this requirement. We cannot hope to make progress until stakeholders—regulators, insurers, industry, clinicians, and patients alike—have the clarity that they need and the data is being collected, reported on, and made available to the public in a meaningful manner.

**FDA Responsibility**

Meaningful patient experience data not only provides sponsors with a better understanding of patient needs and concerns related to their EXPERIENCE receiving the drug during the development process, but also will give patients and providers important data points as they determine treatment pathways that best meet a patient’s unique needs. Understanding a patient’s social and emotional well-being is so fundamental to care that it is a required patient-centered standard in the accreditation process for the Commission on Cancer. Further, in oncology, the Institute of Medicineii concluded “it is not possible to deliver good quality cancer care without using existing approaches, tools, and resources to address patients’ psychosocial health needs.” For manufacturers, tracking patient experience and offering interventions throughout the trial could very likely improve outcomes and may also allow for more efficient trials by improving a patient’s compliance and retention in the trials.

Trial sponsors increasingly recognize their responsibility to measure and record the full patient experience - both physical and psychosocial. In fact, a 2019 studyiii that found 48 of the 59 drug and biologics applications to the FDA in 2018, voluntarily included a table summarizing PED (Kieffer et al., 2019). Of note, seven of the 11 products that did not include a PED table were submitted before Section 3001 of the 21st Century Cures Law went into effect. Approximately 71% of the drugs approved by the FDA voluntarily reported using PED in the review, with patient-reported outcomes (PROs) the most significant source of PED. Although PROs are important, they are only one type of PED and we believe it is critical that FDA help researchers fully understand and navigate the difference in and importance of collecting the full breadth of PED. In addition, we are concerned by the study’s finding that other sources of patient experience data such as studies designed to gather patient input around disease or treatment burden, experiences during or after a clinical trial, patient preference, or other information gleaned from meetings with patient groups such as PFDD meetings and/or summary reports, are considered less frequently by the FDA in the context of drug application review.

Without a doubt, there is interest from both the FDA and manufacturers in understanding a patient’s experience in the clinical trial. We urge the FDA to work with trial sponsors to establish clear guidelines on the screening, identification, reporting, and labeling of PED. We believe that the reauthorization of PDUFA should establish a requirement that such guidelines, reviewer requirements, and communications regulations be in place within one-year post reauthorization.

**Closing the Gap**

As the June 8, 2020 FDA Notice on the next reauthorization of PDUFA sets forth, the current iteration of PDUFA VI includes commitments to enhance regulatory science and expedite drug
development by focusing on enhancing communication between FDA and sponsors during drug development. PDUFA VI also seeks to incorporate the patient’s voice in drug development and advance the use of complex innovative trial designs and model informed drug development. We look forward to the FDA’s published draft guidance to be released by the end of FY 2020 describing approaches to identifying and developing measures for an identified set of impacts (e.g., burden of disease and treatment) which may facilitate collection of meaningful patient input in clinical trials. The guidance promises to address methods to measure impacts in a meaningful way, and identify an appropriate set of measure(s) that matter most to patients. iv

These PDUFA VI Performance Goals and Procedures also reference the future publication of an FDA draft guidance by the end of FY 2021 on clinical outcome assessments that the FDA notes will, as appropriate, supplement the 2009 Guidance to Industry on Patient-Reported Outcome Measures. v As mentioned above in our comments, while PRO’s are important, they are only one type of PED.

Patient Experience Data captures the experiences, perspectives, needs, and priorities related to (but not limited to): 1) the symptoms of their conditions and its natural history; 2) the impact of the conditions on their functioning and quality of life; 3) their experience with treatments; 4) input on which outcomes are important to them; 5) patient preferences for outcomes and treatments; and 6) the relative importance of any issues as defined by patients. vi

Developing clear and uniform guidelines on the screening, identification, reporting, and labeling of the full complement of PED is needed to ensure PDUFA VI’s goal of incorporating the patient’s voice in drug development is achieved. Only when uniformed guidance is developed will the patient voice be able to be fully incorporated in drug development, including in complex innovative trial designs referenced in PDUFA VI.

The Next Reauthorization of PDUFA
The next PDUFA reauthorization should establish clarity and uniformity for stakeholders - regulators, insurers, industry, clinicians, and patients alike – on the incorporation of the patient’s voice in drug development. Specifically, the FDA should publish draft guidance by the end of FY 2023 that sets forth formal requirements on the capture, reporting, and meaningful communication of patient experience data collected in clinical trials.

The Cancer Support Community appreciates the opportunity to share these comments and we look forward to working with the FDA, sponsors, and other stakeholders to ensure that meaningful patient experience data is collected and used to inform the drug development process as Congress intended.

Sincerely,

Kim Czubaruk, Esq.
Senior Director, Policy and Advocacy
Cancer Support Community
\[\text{i} \quad \text{21st Century Cures Act (Title III, section 3001, Pub. L. 114-255), as amended by the Food and Drug Rehabilitation Act of 2017 (section 605, Pub. L. 115-52) (FDARA).}\]

\[\text{\large ii} \quad \text{Institute of Medicine. (2008). \textit{Cancer care for the whole patient}. Washington, DC: Author.}\]


\[\text{\large iv} \quad \text{PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2018 Through 2022. Retrieved August 20, 2020, from \url{https://www.fda.gov/media/99140/download}}\]

\[\text{\large v} \quad \text{PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2018 Through 2022. Retrieved August 20, 2020, from \url{https://www.fda.gov/media/99140/download}}\]

\[\text{\large vi} \quad \text{21st Century Cures Act (Title III, Section 3002(c)).}\]