November 29, 2021

Janet Woodcock, MD
Acting Commissioner
Food and Drug Administration
5630 Fishers Lane, Rm 1061
Rockville, MD 20852

Via Electronic Submission: https://www.regulations.gov

Re: Docket No. FDA-2020-D-2316 for "Benefit-Risk Assessment for New Drug and Biological Products; Draft Guidance for Industry."

Dear Acting Commissioner Woodcock,

The Cancer Support Community (CSC), an international nonprofit organization that provides support, education, and hope to cancer patients, survivors, and their loved ones, appreciates the opportunity to comment on the Food and Drug Administration's (FDA or Agency) draft Guidance titled *Benefit-Risk Assessment for New Drug and Biological Products; Draft Guidance for Industry* ("draft Guidance").

As the largest direct provider of social and emotional support services for people impacted by cancer, CSC has a unique understanding of the cancer patient experience. 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.

Patient Experience Data

Passage of the *21st Century Cures Act* in 2016 and the *Food and Drug Administration Reauthorization Act (FDARA)* in 2017 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. PED 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 (21st Century Cures, 2016). Understanding, including, and embracing PED as an essential component of a drug's benefit-risk assessment is necessary to ensure patients' experiences, perspectives, needs, and priorities are meaningfully integrated into the drug development and approval process as Congress intended.

Benefit-Risk Framework Generally

When assessing the benefit-risk of a drug or biologic (hereafter "drug"), the FDA considers the therapeutic context in which the drug will be used, the evidence submitted, the uncertainty about the drug's benefit and risks, and the FDA's regulatory options to reduce uncertainties and manage risk (FDA, 2021). CSC agrees with the FDA's recognition that **PED is a source of evidence** submitted to inform the FDA's understanding of the benefits and risks of a drug in a pre-market application and post market setting. Similarly, we support and appreciate the FDA's recognition that in its Benefit-Risk Framework used to identify, assess, and communicate important benefit-risk assessment considerations, evidence, and thus PED, is relevant to the benefits and risks of a drug, as well as to the analysis of the condition and current treatment options**.** Acknowledging the integral role PED plays as a source of evidence to be considered in benefit-risk assessment is essential to achieving Congress' intent in *21st Century Cures* to heighten the importance of PED.

Patient Experience Data as a Consideration in Premarket Benefit-Risk Assessment

The draft Guidance echoes a fundamental premise consistently asserted by CSC on behalf of patients – namely that patients are the experts in their disease. PED, like other sources of evidence, are extremely informative to the benefit-risk assessment. However, to be a consistent and meaningful source of evidence in benefit-risk assessment, sponsors must know **what** PED to collect, **how** to uniformly capture and report it, and be **required** to do so as part of their new drug application.

We agree that the use of a methodologically-sound and fit-for-purpose data collection tool to collect PED can provide direct evidence as to the benefit-risk assessment of a drug and its importance to patients. We also assert the same is true for all evidence submitted to inform the FDA's benefit-risk assessment, not solely PED. Equally important, this acknowledgement supports CSC's call for formally defining what PED sponsors must collect and how they uniformly capture and report it.

Earlier in the draft Guidance, the FDA references use of the integrated review process and template developed by the Center for Drug Evaluation and Research (CDER) in 2019. On August 26, 2019, CSC commented on the integrated review process and accompanying template (CSC, 2019). We expressed concern then that the lack of a requirement for sponsors to collect PED and the failure to define the scope of the PED required to be collected leaves valuable information that is only obtainable from patients, unrealized. The FDA confirms in this draft Guidance that **PED can inform nearly every aspect of benefit-risk assessment** throughout the drug lifecycle. While we applaud the FDA's recognition of PED's breadth of impact, we reiterate the same concerns expressed in our August 26, 2019, comments - **PED that is not collected cannot serve as a source of evidence.**

CSC understands and appreciates the FDA's need to balance patients' perspectives with the overall benefit-risk assessment of a drug for the patient population. In working to achieve this balance, we support and applaud the FDA's stated commitment to **carefully weigh and consider** patient perspectives. Importantly, this commitment to carefully weigh and consider patient perspectives first demands the collection and submission of PED.

Collecting Patient Experience Data During Development to Inform Benefit-Risk Assessment

The draft Guidance acknowledges the importance of PED in a drug development program and in connection to benefit-risk assessment broadly. While a positive step, the FDA **encouraging** sponsors **considering the collection** and utilization of PED as part of their evaluation of effectiveness or safety to have early interactions with the Agency to discuss research design, fails to convey the priority and respect deserving of evidence that can help identify unmet patient needs, define target populations, and help identify endpoints that measure or predict clinical importance to patients (FDA, 2021). Similarly, Congress' intent to heighten the importance of PED will not be achieved by sponsors merely **considering** the collection of PED.

FDA acknowledges that developing a patient-focused outcome measurement approach to clinical outcome assessment (COA) selection and/or clinical trials is a primary component of this guidance series. As repeated throughout these comments, CSC has been, and continues to be, a leading voice for establishing formal requirements on what and how to collect PED. While we fully support the development of approaches to measure patient-focused outcome measures, without a requirement obligating sponsors to collect PED and without formalizing requirements for what types of PED to collect, tools to measure PED will have limited benefit.

The draft Guidance correctly recognizes the value of patient preference information (PPI), noting its usefulness to sponsors throughout drug development including for purposes of informing the therapeutic context, identifying endpoints, and informing benefit-risk assessment. It is regrettable, therefore, that despite lauding these potential benefits of PPI, the "if available" qualifier reduces the likely availability of PPIs as a consideration to inform FDA's assessment of a drug's efficacy and safety to the patient population.

Five and four years, respectively, have passed since *21st Century Cures* in 2016 and the *Food and Drug Administration Reauthorization Act (FDARA)* in 2017 heightened the importance of collecting PED that includes both the physical and psychosocial impacts of a condition, therapy, or clinical investigation. Despite this lapse of time, the draft Guidance is a reminder that sponsors still have no obligation to collect, use, or submit PED to inform the FDA's risk-benefit analysis of new drugs. This remains true notwithstanding the FDA's acknowledgment that PED can inform nearly every aspect of the FDA's benefit-risk assessment throughout the drug lifecycle.

Benefit-Risk Assessment in the Postmarket Setting

While the draft Guidance makes no specific reference to PED in connection to the postmarket setting, cancer patients' experiences, perspectives, needs, and priorities continue to evolve following the approval of a drug. In addition, the postmarket period may present the first opportunity to conduct a benefit-risk assessment of a drug that is being used by a heterogenous patient population. For these reasons, we encourage the collection and consideration of PED in the postmarket setting to serve as postmarket evidence to inform benefit-risk assessment.

Conclusion

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 PED serves as

important evidence in the FDA's benefit-risk assessment for new drugs and also has the opportunity to inform postmarket benefit-risk assessment. Should you have any questions or would like to discuss these comments in more detail, please reach out to Kim Czubaruk at kczubaruk@cancersupportcommunity.org.

Sincerely,

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References

21st Century Cures Act, Title III, section 3001, Pub. L. 114-255 (2016). [As amended by the Food and Drug Rehabilitation Act of 2017, section 605, Pub. L. 115-52 (2017).]

Cancer Support Community. (2019, August 26). Comments on Docket No. FDA-2019-N-2012 for "New Drugs Regulatory Program Modernization: Improving Approval Package Documentation and Communication."

Food and Drug Administration. (2021). Benefit-Risk Assessment for New Drug and Biological Products; Draft Guidance for Industry. Retrieved at https://documentcloud.adobe.com/link/review?uri=urn:aaid:scds:US:dccb37ec-2f37-468a-a412-8eb86976fb56