May 18, 2018

Scott Gottlieb, MD
Commissioner for Food and Drugs
U.S. Food and Drug Administration
10903 New Hampshire Ave.
Silver Spring, MD 20993


Dear Dr. Gottlieb,

On behalf of the Cancer Support Community (CSC), an international nonprofit organization that provides support, education, and hope to cancer patients, survivors, and their loved ones, we appreciate the opportunity to attend the workshop for and provide comments on the Patient-Focused Drug Development (PFDD): Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data; Public Workshop; Request for Comments (Guidance, or “Guidance on guidance”). The development of this Guidance is critical to successfully incorporating patient experience data into the drug development process to meet the requirements of section 3002 of the Cures Act (Pub. L. 114-255) and CSC is thankful for the opportunity to provide comments on this process.

CSC has a unique understanding of the patient experience as we serve patients and caregivers through a network of 175 locations, including CSC and Gilda’s Club centers, health-care partnerships, and a dynamic online community. Overall, we deliver more than $50 million in free, personalized services each year to individuals and families affected by cancer nationwide and internationally. The following comments reflect what we have learned from that frontline experience, captured scientifically through our Cancer Experience Registry, which serves as the cornerstone of our research efforts. Our Registry of over 13,000 individuals allows us to measure what matters most to patients and caregivers so that we can translate these findings into policy recommendations.

We commend the Food and Drug Administration (FDA or the Agency) on its efforts to include the voice of the patient in drug development and respectfully submit the following comments for consideration in the development of the forthcoming “Guidance on guidance.” CSC has taken advantage of numerous opportunities to submit public comments and to partner with the FDA to discuss the importance of measuring the full patient experience in clinical trials. In fact, the CSC
worked with the Congress to expand section 3002 (Pub. L. 114-255) to ensure that the definition of patient experience data explicitly include psychosocial measures along with physical measures. We firmly believe that failing to measure the psychological impact of any given intervention on the patient will fail to capture the true patient experience. We believe this “Guidance on guidance” creates an opportunity to map out a thoughtful process that will result in a clear framework for manufacturers to operationalize the integration of psychosocial data as well as the requisite support services in their protocols.

CSC believes all stakeholders must be engaged in this process, including patient advocacy organizations, individual patients, manufacturers, and the FDA, to map out practical metrics to capture patient experience data. CSC requests that the forthcoming guidance be very clear to manufactures as to how they can best identify and incorporate patient experience metrics into clinical trial protocol and why doing so is in the best interest of both the patient and industry.

**Background**

The social and emotional impact of cancer is undeniable. What is perhaps more compelling to the FDA is that distress related to cancer is in part, caused by the treatment a patient receives. Distress can negatively impact a patient’s adherence to a clinical trial, to their medications, and ultimately their path towards health. Nearly half of all cancer patients experience moderate levels of distress (Institute of Medicine, 2008) which has been defined as emotional, social, spiritual, or physical pain or suffering (National Cancer Institute, n.d.). “Cancer patients may have trouble coping with their diagnosis, physical symptoms, or treatment” (National Cancer Institute, n.d.) ultimately leading to such distress.

CSC has found that when patients experience distress that interrupts their work, family, or school life, they have an 86% probability of developing a clinical diagnosis of depression (Miller et al., 2016). Patients with depression can have lower rates of treatment adherence (Buzaglo et al., 2014), higher health care utilization rates, and higher annual health care costs (Jeffrey et al., 2011).

Patients who have the full range of their needs assessed and met are more likely to maintain enrollment in a study, adhere to treatment, and ultimately experience improved outcomes—as they define them. For example, studies have shown that breast cancer patients who were engaged in social and emotional interventions had significant psychological and physiological benefits over those who do not, including survival advantages (1.5 years), reduced risk of recurrence (45%), as well as decreases in anxiety, and fewer symptoms and toxicities from cancer treatment (Andersen et al., 2010, 2008, 2004).

As we explore opportunities to improve recruitment and retention in the clinical trial and better involve patients into drug development, we must account not only for biomedical endpoints and outcomes in trials, but for the full range of issues that impact patients. Information about levels of experiential distress caused by a treatment should be collected, reported on, and used as part of shared decision-making process both for the benefit of the patient and their family but also for future trial design and intervention. Simply put, incorporating patient experience metrics into
Trial design and developing treatments that truly meet patient needs will not only dramatically improve the quality and relevance of the data that is collected but is also simply in the best interests of patients. The intent of measuring patient experience as part of a clinical trial is to better understand the patient response to each arm of a trial, similar to documenting physical response to the interventions in each arm of a trial.

The below comments are in response to the questions that were outlined in the call for comments as well as the questions that were raised by the FDA at the March 19, 2018 public workshop.

1. **Defining the scope of the proposed draft guidance: What questions should the FDA ask in the forthcoming guidance?**

   CSC would like the FDA to take this opportunity to specifically recommend how to capture data that reflects the full patient experience by promoting the collection of psychosocial patient experience measures in addition to the traditional physical experience measures. To do so, we encourage the FDA to ask the following questions in the forthcoming guidance:
   
   - What type of patient experience/psychosocial impact measures are the most useful to understand the full impact of a therapy for a defined patient population?
   - Who can collect and submit patient experience data?
   - What type of patient experience data is considered valid (i.e., registry data, surveys, electronic health record data, etc.?)
   - How will the FDA prioritize the type of patient experience data that is collected and submitted (to prevent wasting of resources)?
   - How well do drug labels reflect what patients want to know about a therapy?
     - Is there a meaningful void in the patient experience in existing labels that should be addressed?
   - Will the inclusion of this patient experience data fill an unmet need/address unanswered questions?

2. **Developing the proposed draft guidance**

   In the forthcoming guidance it would be useful to list the formal and informal opportunities for patients and the patient advocacy community to be involved in the PFDD process. Patient advocacy organizations, such as CSC, have a wealth of resources and tools that can be used to understand the patient experience and organizations look forward to collaborating with the FDA. When outlining opportunities, please prioritize and explicitly state opportunities for collaboration and expertise from the patient community.
3. **Submitting the proposed draft guidance to the FDA (including the process and format)**

To ensure a high response to the proposed draft guidance, the FDA should share as much information as possible on its website. It would also be useful for the FDA to share a suggested template for guidances with a disclaimer that there is room for variability as disease areas differ as do the sophistication and capability of patients and patient advocacy organizations.

In closing, it is deeply encouraging to us that the FDA recognizes that what matters to patients is more than the physical and biological impacts of treatments but also the equally and often more complicated social, emotional, and logistical implications of a given treatment. It is incumbent upon the FDA to implement the statutorily expanded definition of patient experience data to include both physical and psychological impacts of a condition, therapy, or clinical investigation. This “guidance on guidance” provides an opportunity for the FDA to prioritize engagement of the patient advocacy community and to facilitate dialogue directly with industry to understand what it needs to better collect and share patient experience data through patient-focused drug development. CSC recommends that there be a dedicated effort to examine how to best incorporate psychosocial measures into the clinical trial process to capture the full patient experience as the Food and Drug Administration Reauthorization Act of 2017 suggests. The Agency would be well served to partner with all stakeholders, to include behavioral health experts, patient advocacy organizations, academia, and industry sponsors who are already collecting psychosocial and patient preference data to develop guidance that clearly maps out how to identify and incorporate patient experience metrics into clinical trials.

CSC stands ready to assist the agency in meaningfully integrating the expanded definition of patient experience data into all forthcoming guidances. CSC has long believed that the private sector should lead in the collection of patient experience data as much as the FDA should lead in requiring sponsors to submit patient experience data and we offer the opportunity to convene both parties to identify areas of collaboration.

Sincerely,

Linda House, MSM, BSN, RN
President
Cancer Support Community
References


