September 10, 2018

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

Re: Docket No. FDA-2018-D-1893: Patient-Focused Drug Development: Collecting Comprehensive and Representative Input (Guidance 1); Request for Comments

Dr. Gottlieb,

On behalf of the Cancer Support Community (CSC), an international nonprofit organization that provides support, education, and hope to people impacted by cancer, we appreciate the opportunity to submit the following comments in response to: Patient Focused Drug Development: Collecting Comprehensive and Representative Input. CSC applauds the U.S. Food and Drug Administration (FDA or Agency) for its efforts to systematically integrate patient experience and patient preference data into the drug development and approval processes and we are encouraged to see the integration of the expanded definition of patient experience data which was amended by the FDA Reauthorization Act of 2017 to include psychosocial as well as physical impacts of a condition, therapy, or clinical investigation/trial.

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. Each year, CSC serves more than one million people affected by cancer through its network of over 45 licensed affiliates, more than 170 satellite locations, and a dynamic online community of individuals receiving social support services. Overall, we deliver more than $50 million in free, personalized services each year to individuals and families affected by cancer nationwide and internationally.

Additionally, CSC is home to the Research and Training Institute (RTI)—the only entity of its kind focused solely on the experiences of cancer patients and their loved ones. The RTI has contributed to the evidence base regarding the cancer patient experience through its Cancer Experience Registry, various publications and peer-reviewed studies on distress screening, and the psychosocial impact of cancer, and cancer survivorship. This combination of direct services and research uniquely positions CSC to provide valuable patient and evidence-informed feedback to the FDA.

As noted in in the Draft Guidance, patient input not only informs the clinical context and provides insights to frame the assessment of benefits and risk but it can also serve as a direct
source of evidence for use in clinical studies in investigational therapies. Clinical trial design, trial endpoint selection, regulatory reviews, and post-market surveillance should all include meaningful patient feedback and patient experience data. Ultimately, this information can be incorporated into drug labeling to better inform decisions by patients, their loved ones, and their health care team.

This Guidance is not binding on FDA or the public. All components are discussed in the context of “should,” or a recommendation of what stakeholders might consider doing, but nothing is a requirement. If FDA policy is that patient perspectives and patient preferences are crucial to determining the safety and efficacy of a product then the Agency should require that innovators capture this information throughout the drug discovery and development process.

**Value Patient Expertise**

Patients are the foremost experts in their disease including their values, needs, and preferences. They should be continuously consulted and treated as such. To formally acknowledge the role that the patient should play in PFDD, we recommend the following edits to this Guidance:

- Page 7, Line 220: Explicitly state the importance of the role of the patient by editing to read as “…to ensure important clinical outcomes as defined by the patients are studied.”
- Page 11, Line 359: When referring to subject matter experts, please include patients in the list of potential subject matter experts. Please edit to read as “…subject matter experts (e.g., patients, clinicians, social scientists, etc.).”
- Page 7, Line 228: Edit to read “Patients are the foremost experts in their own experience of their disease…”
- Page 13, Line 429: Define what is meant by “Insight.”
- Page 13, Line 438: It is dismissive of the experiences of all patients to screen feedback based on cognitive function. While such feedback may need to be supplemented with additional data, patients living with a disability should not be precluded from providing information about their experience.

**Engage Patients Early and Continuously**

For drug development to be truly patient-focused, patients must be involved throughout the entire process. As such, they should be meaningfully engaged even before the research objectives and questions are developed. We recommend added a preliminary step to the chart on Page 11 to gather patient input, and ask patients what matters to them before moving on to Step 1 (define the research objective(s) and questions).

It is incumbent upon the FDA, industry, academic institutions, members of the health care team, patient advocacy organizations, and other stakeholders to consistently and meaningfully seek robust patient feedback and patient experience data at all points along the research and care continuum. It is essential to involve patient experts at every step of the process so that no assumptions are made as to what a patient may or may not prefer. We recommend that you edit the example on Page 12, Lines 371-386, to recommend asking patients how they would prefer to be surveyed rather than assuming that they would prefer one-on-one interviews over the telephone. This requires adding a step of patient engagement, but this is essential.
Meaningfully Incorporate Psychosocial Data
We acknowledge that the FDA has made progress in expanding the definition of patient experience data to include psychosocial (as well as physical) impacts and we ask the Agency to ensure progress is made to meaningfully incorporate this change into all PFDD efforts. As such, CSC recommends that the FDA routinely and explicitly state “psychosocial” throughout the guidance as well as in future Guidance issued. The following areas in the draft Guidance should be updated by adding “psychosocial”:

- Page 2, Line 58: Edit to read: “…Guidance 1, gathering information about what aspects of symptoms, psychosocial and physical impacts of their disease, and other issues are important to patients.”
- Page 6, Lines 194-202: Psychosocial impacts should be explicitly listed as one of the potential impacts of the disease and its treatment plan.
- Page 23, Clinical Characteristics listed in Figure 3: Explicitly list the “range of social and emotional impact.”
- Page 7, Line 237: Edit “Patient experience data can be used to help identify unmet medical needs and important clinical outcomes to be studied…” Remove “medical” and “clinical” to read “Patient experience data can be used to help identify unmet needs and important outcomes to be studied…”

Incorporate Data Sources Beyond Clinical and Medical Data
The draft Guidance is written in a way that mainly focuses on the clinical and medical experiences of patients and should be amended to put equal emphasis on the social and emotional experiences of patients. CSC recommends that the FDA revise this draft Guidance by adding proportionate focus on the psychosocial aspects of care. The following are some specific areas where the Guidance may be amended, however this should be addressed throughout the Guidance:

- Page 6, Line 211: To expand beyond the medical needs, we recommend that you edit this bullet to read as “Views on medical, health, social, emotional, and practical needs and currently available treatment options”
- Page 7, Line 220: This Guidance should not be limited to focus on clinical outcomes. CSC recommends that the FDA modify this sentence to expand beyond clinical outcomes, and to add “…outcomes as defined by the patients”
- Page 6, Line 197: Change “chief complaints” to “chief burdens” to recognize the significant impact that such bothersome signs/symptoms have on patients

Amend Research Methodology
It is important to recognize that qualitative, quantitative, and mixed methods research methodologies are equally valid and important. There are opportunities in the Guidance to reinforce this notion including:

- Page 14, Line 491: Edit to include qualitative methodology experts to the list of subject matter experts
- Page 14, Line 502: Edit to read as “…sampling approach could be appropriate.”
- Page 21, Lines 608-616: We believe that the description of qualitative methods is not accurate. For example, the Guidance states that sample size determination is often less
formal. Sample size determination may not be based upon statistical power, however qualitative research methodology relies upon evidence-based strategies to ensure research rigor. In qualitative work, researchers select an initial sample size based on their study topic and population of interest and explore the experiences of individuals in the sample until saturation has been met, or the point at which no additional data collection is needed (Padgett, 2008; Glaser & Strauss, 1967). The Guidance also points out that sample size determination for qualitative studies is usually subjective, though there is some Guidance in the literature. There is a robust literature base regarding qualitative methodology and the Guidance does not accurately or appropriately depict the breadth and depth of qualitative research methodology, particularly as compared to the description of quantitative research in the Guidance.

- Page 15, Figure 4: The figure depicting “General Steps for Data Analysis in Qualitative Research does not appropriately depict the scientific rigor behind qualitative data analysis. For example, there are various methods of coding and analyzing data and there is a general lack of content in this Guidance regarding methods to enhance rigor such as member checking, debriefing, triangulation, negative case analysis, the creation of an audit trail, and the acknowledgement of researcher reactivity.
- Page 24, Line 720-721: The CSC offers ourselves as a resource to the FDA to identify other methods of collecting and analyzing patient experience data.
- Page 21, Line 610 states that “For qualitative studies, sample size determination is often less formal....” This is not true and stating as such undermines the value of qualitative data.

Incorporate Patient Experience Registries
Patient registries contain a variety of data, both qualitative and quantitative, and should be leveraged to complement primary data used in PFDD. The Cancer Experience Registry has robust data on the cancer patient experience of over 13,000 individuals impacted by cancer. This registry, as well as others, offer meaningful insight into the cancer patient experience that likely is not captured outside of the registry.

Incorporate Distress Screening into PFDD
Conducting distress screening and collecting patient-experience data is good clinical practice, and patients enrolled in clinical research should be afforded such protections. The Belmont Report, which is referenced in the World Health Organization Good Clinical Practices (WHO-GCPs) document states, “[m]any kinds of possible harms and benefits need to be taken into account. There are, for example, risks of psychological harm, physical harm, legal harm, social harm and economic harm and the corresponding benefits. While the most likely types of harms to research subjects are those of psychological or physical pain or injury, other possible kinds should not be overlooked.” Utilizing tools such as the Cancer Support Source to screen for distress is good clinical practice and will also provide an additional measurement of the patient experience that may not be captured elsewhere in a trial.

Utilize Electronic Health Records as Data Source
Data captured in electronic health records such as reports from distress screenings should be considered as data sources to be used in PFDD. We recommend adding “Electronic Health Records” to Table 5 on Page 31-32.
Conclusion
In closing, the Cancer Support Community continues to support the FDA’s efforts to promote the
collection of patient experience data throughout the drug development process and looks forward
to the opportunity to collaborate with the Agency, drug manufacturers, and patient advocacy
organizations to further this endeavor. We would like to commend the FDA for working to bring
together representative input to ensure that patient experience data is an integral part of the future
of drug development, and we thank the FDA for the opportunity to submit these comments. To
ensure that stakeholder input is meaningfully shared and the Agency has an opportunity to
provide guidance to stakeholders, we ask that the FDA give prescriptive guidance as to how
stakeholders can and should engaged with the FDA early and often. Specifically, we would like
the FDA to provide details on: who at the FDA stakeholders should contact, what forms of
interactions with the Agency should be requested, what do stakeholders need to bring with them
to meetings with the Agency to make the interactions productive, and how frequently should
interactions take place. Providing this level of detail will help stakeholders prepare for
productive interactions with the Agency. As always, CSC stands ready to serve as a resource to
the FDA as we collectively work to protect patients and elevate their voices throughout our
regulatory processes.

Sincerely,

Elizabeth F. Franklin, LGSW, ACSW
Executive Director, Cancer Policy Institute
Cancer Support Community Headquarters

References


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i The National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research. Belmont
Report ethical principles and guidelines for the protection of human subjects of research. Retrieved from: