



Enhancing Integration of Patient Perspective Data in the Drug Development Process

Proposal for PDUFA VI

For your consideration, below is a proposal from FasterCures, designed to help improve and enhance the integration of patient perspective data into the drug development and regulatory approval process. We recognize that this proposal is being submitted in connection with PDUFA VI, however we think that optimal impact will be achieved if this approach is applied not just to drugs but to the development and review of all medical products. Our goal is for this language to be included in the PDUFA VI commitment letter and to facilitate that, we have modeled the language after analogous proposals outlined in the PDUFA V commitment letter.

I. Advancing the Science of Patient Input

- a. Patient Perspective Data Defined – Patient perspective data means information gathered from the perspective of patients and/or caregivers about their experiences, expectations, and tolerance with respect to a disease or condition that includes:
 - i. Symptoms experienced
 - ii. Chief complaints (most significant or serious symptoms or signs of illness or dysfunction that cause the patient to seek health care)
 - iii. The burden of managing and/or living with a disease
 - iv. Impacts on activities of daily living and functioning
 - v. Strengths and weaknesses of currently available therapeutic options, including side effect profiles
 - vi. Unmet medical need
 - vii. Poorly tolerated side effects that may render a therapeutic option unacceptable
 - viii. Disease natural history, severity, and chronicity
 - ix. Minimum expectations of benefits
 - x. Maximum tolerable harms or risks that a patient might be willing to accept
 - xi. Which attributes, side effects, outcomes or features of a medical product are important to them
 - xii. The relative importance of different outcomes or features of a medical product
 - xiii. The tradeoffs patients are willing to make
 - xiv. Attitudes toward uncertainty
 - xv. Decisions regarding their care that patients might encounter, or
 - xvi. Other matters important to patients

- b. To enhance the science of patient input and better integrate patient perspective data into the medical product development and regulatory review processes, FDA will conduct the following activities:
- i. Develop a working group, consisting of a substantial number of relevant experts from CDER, CBER, CDRH, and ORSI staff, with appropriate expertise to evaluate different quantitative and qualitative methods and to explore the practical application of scientific approaches and best practices for the integration of patient perspective data into medical product development and regulatory review. The evaluated methods should include but not be limited to, methods catalogued by the Medical Device Innovation Consortium in the appendix to its Patient Centered Benefit-Risk Framework Report. The working group will also evaluate and explore limitations of the identified methodologies in supporting development, regulatory, and post-marketing decisions related to medical products.
 - ii. By the end of FY 2018, hold a public meeting engaging stakeholders in discussing current and emerging scientific approaches and methods to collect and obtain patient perspective data, and to facilitate stakeholder feedback and input regarding the use of patient perspective data in FDA's regulatory review process.
 - iii. Considering feedback and input received through the public meeting, publish a draft guidance document for comment describing FDA's intended approach to the use of patient perspective data in the FDA's regulatory review process by the end of FY 2019. This guidance will promote a better understanding and more consistency among Agency, industry, patient groups, and other stakeholders regarding patient perspective data and its role in medical product development and regulatory decision-making.
 - iv. Complete the final guidance describing FDA's intended approach to the use of patient perspective data in FDA's regulatory review process (or revised draft guidance, if appropriate) within 1.5 years of the close of the public comment period.