Patient and Consumer Stakeholder Meeting on MDUFA IV Reauthorization
November 30, 2015, 9:00 – 11:00 AM
FDA White Oak Campus, Silver Spring, MD
Building 31, Great Room Section C

Purpose

To provide a status update on the ongoing MDUFA IV negotiations, plan for future stakeholder meetings, and obtain stakeholders' views on the focus topics of patient engagement and scientific input.

Participants

FDA

Malcolm Bertoni	Office of the Commissioner (OC)
Marc Caden	Office of Chief Counsel (OCC)
Jonette Foy	Center for Devices and Radiological Health (CDRH)
Sonja Fulmer	CDRH
Louise Howe	OCC
Heather Howell	CDRH
Aaron Josephson	CDRH
Sheryl Kochman	Center for Biologics Evaluation and Research (CBER)
Thinh Nguyen	Office of Combination Products (OCP)
Kathryn O'Callaghan	CDRH
Prakash Rath	Office of Legislation (OL)
Anindita Saha	CDRH
Don St. Pierre	CDRH
Darian Tarver	OC
Shannon Thor	OC
Jacquline Yancy	CDRH
Barb Zimmerman	CDRH

Stakeholders

Alexandra Bennewith	United Spinal Association
Cynthia Bens	Alliance for Aging Research
Paul Brown	National Center for Health Research
Ryne Carney	Alliance for Aging Research
Diane Dorman	dDConsulting
Christin Engelhardt	National Coalition for Cancer Survivorship
Mark Fleury	American Cancer Society Cancer Action Network
Eric Gascho	National Health Council
Lisa Goldstein	American College of Cardiology
Marisol Goss	AAOS
Catherine Hill	American Academy of Neurosurgery

Maureen Japha	FasterCures
Bennie Johnson	JDRF
Andrea Lowe	Society for Women's Health Research
Anqi Lu	Pew Charitable Trusts
Lisa M. Tate	Healthy Women
Paul Melmeyer	National Organization for Rare Disorders
Ben Moscovitch	Pew Charitable Trusts
Brian Smith	Research!America
Jessica Tyson	Avalere Health
Jessica Foley	Focused Ultrasound Foundation
Charles Cascio	American College of Cardiology

Meeting Start Time: 9:00 am

FDA welcomed stakeholders, briefly reiterated the role of stakeholder input during MDUFA negotiations and provided a summary of the topics discussed at the last MDUFA negotiation meeting.

The most recent negotiation meeting with Industry was held on November 18, 2015. The meeting included presentations of proposals by both Industry and FDA. Both Industry and FDA agreed that the program was in a good place and some process improvements were needed as evidenced by the overlap in some of the proposals presented by both sides. FDA's package of proposals included investments in strengthening patient input and using real world clinical experience and registry data. The minutes of the most recent meeting with Industry are posted on FDA's website. FDA and Industry agreed to review each proposal and submit clarifying questions in advance for discussion at the next meeting on December 15, 2015.

For the focus topic, FDA presented progress and ongoing efforts related to implementation of FDASIA Section 1137, patient engagement, the science of patient input, and related MDUFA III commitments.

FDA presented information regarding how FDA uses patient input to satisfy the requirements of FDASIA section 1137 and how such input is used in premarket reviews. Specifically, FDA discussed the items we committed to accomplish in MDUFA III regarding patient safety and risk tolerance. FDA has implemented its benefit risk guidance, which includes patient tolerance for risk and perspective on benefit in premarket reviews. Also, FDA held a public meeting in 2013 to better understand how to characterize patient perspectives, with a focus on disease severity and unmet medical needs. In addition, FDA has increased utilization of patient representatives as consultants to FDA to provide patients' views.

FDA updated stakeholders on additional activities CDRH has undertaken to promote patient engagement and invest in the science of patient input. Specifically, the agency highlighted the recent establishment of the Patient Engagement Advisory Committee

(PEAC), which will help assure that the needs and experiences of patients are incorporated in FDA's work. FDA stated that this group will also serve as a resource to the Agency, sharing expertise related to patient and caregiver experiences, needs, and activities of the patient community related to safe and effective medical product use.

FDA highlighted two areas of activity under the science of patient input: the Patient Preference Initiative, and expanding use of Patient Reported Outcomes (PROs). Under the Patient Preference Initiative, FDA conducted a demonstration case study to better understand how obese patients think about tradeoffs between potential benefits and risks of a variety of weight-loss treatment options. The data from this study was considered by the Agency in its benefit-risk assessment of a new weight-loss device that was approved this year, the first weight-loss device approved since 2007. The data from the study is also being used in earlier stages of device development/review to inform design of clinical studies for new weight-loss devices. FDA also issued draft guidance documents on patient preference: one addressing IDEs that include clinical study protocols, and another explaining what patient preference information is and how it can be used in benefit-risk assessments for certain marketing applications (PMAs, HDEs and de novo classifications).

FDA highlighted the increasing number of PROs included in submissions reviewed by CDRH since FDA issued a guidance document about PROs in 2009. In a retrospective analysis, FDA found that 20 or fewer submissions per year included PROs prior to 2009; however, after issuance of the guidance, that number increased substantially every year, with over 120 submissions including PROs in 2014. Further analysis identified more than 500 premarket applications that included PROs. FDA reiterated that CDRH does not have targeted resources to review PROs, and it relies on current staff to review this component of submissions. FDA stated that it is seeking targeted user fees to support reviews of PROs.

Stakeholders presented on the focused topic.

Three patient advocacy groups presented their thoughts about integrating patient perspectives into FDA's regulatory decision making process. Specifically, these groups continued to emphasize that patient perspective information does not only include patient preference. One group outlined a range of potential applications for "patient perspective data," which may serve to help define the term for purposes of revising the draft guidance. This included general applications such as symptoms experienced, chief complaints (most significant or serious symptoms that cause individual to seek health care), burden of managing or living with a condition, impacts on daily living and functioning, strengths and weaknesses of currently available therapeutic options, experience of progression, severity and chronicity, views on unmet medical need, minimum expectations of benefits, maximum tolerable harms or risks, acceptable tradeoffs, attitudes toward uncertainty, decisions regarding care that patients might encounter. The group also outlined several potential product-specific uses, such as most important attributes, outcomes, or features of a medical product, and relative importance of such features.

The stakeholders also noted the importance of not excluding caregiver/proxy perspective, not just for scenarios outlined in the draft guidance where a patient cannot provide their perspective directly, but also for continuity of care in progressive conditions. The stakeholders inquired about having a clear pathway defined for non-product sponsors to submit information to FDA. The stakeholders expressed support for the establishment of the PEAC, but cautioned the Agency to ensure the advisory committee deliberations not be structured in isolation of other considerations before the Agency. The stakeholders expressed concern about CDRH authorities related to the expedited review of diagnostics, including LDTs, for the rare disease community. The stakeholders called for clearer distinction between patient reported outcomes and patient preference terminology. The stakeholders discussed the benefits of including patient preference studies in the labeling. The stakeholders expressed concerns with resources being available to sustain the work being done, as well as to improve initiatives such as PROs, and to enhance the use of real world evidence to support premarket and postmarket activities.

The next patient and consumer stakeholder meeting is scheduled for Friday, December 18, 2015. The focused discussion topic will be use of real world evidence.

End 10:47am