Patient and Consumer Stakeholder Meeting on MDUFA IV Reauthorization
December 18, 2015, 9:00 – 11:00 AM
FDA White Oak Campus, Silver Spring, MD
Building 31, Great Room Section A

Purpose

To provide a status update on the ongoing MDUFA IV negotiations, plan for future stakeholder meetings, and obtain stakeholders' views on the focus topic of the use of clinical experience information, or "real world" evidence.

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)
Danica Marinac-Dabic	CDRH
Thinh Nguyen	Office of Combination Products (OCP)
Kathryn O'Callaghan	CDRH
Prakash Rath	Office of Legislation (OL)
Anindita Saha	CDRH
Greg Pappas	CDRH
Don St. Pierre	CDRH
Darian Tarver	OC
Shannon Thor	OC
Jacquline Yancy	CDRH
Barb Zimmerman	CDRH

Stakeholders

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
Brian Fiske	Epilepsy Foundation
Eric Gascho	National Health Council
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
Andrew Sperling	National Alliance on Mental Illness
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 December 15, 2015. At that meeting, FDA provided more detailed analysis of numerous proposals put forth by both FDA and Industry, including:

- strengthening the device premarket review infrastructure, which FDA believes is a necessary foundation for all the other improvements that could be made to the program;
- FDA's innovation proposals for 1) leveraging real world evidence for device evaluation and 2) improving the predictability, consistency and "quality journey" for submissions with patient centered data;
- several process enhancement proposals regarding pre-submission consultations, de novo classifications, Clinical Laboratory Improvement Amendments (CLIA) waivers, and mechanisms for addressing workload uncertainty.

The minutes of the December 15 meeting with Industry are posted on FDA's website. FDA and industry agreed to establish working groups on certain topics before the next meeting scheduled for January 20, 2015.

For the focus topic, FDA presented the use of Clinical Experience or "Real world evidence" (RWE).

FDA presented information regarding why building a national evaluation system for medical devices is expected to increase patient safety, provide faster access to beneficial new technologies, and facilitate better quality care for patients. FDA emphasized that further development of this system will help bring devices, including life-saving devices, to patients and healthcare professionals more quickly, as well as help us reduce time to understand problems with devices in an effort to further improve the safe and effective use of those devices. A national evaluation system would also facilitate more personalized medicine and a learning healthcare system by providing a better

understanding of which patient groups may benefit most, and how to optimize medical care and choose the best options for particular patients.

FDA emphasized that the goal of the development of the national system is to evaluate the entire life cycle of the device, not just postmarket activity. FDA briefly discussed the strategy to link data registries as outlined in two white papers that were published in 2012 and 2013.

FDA presented some of the foundational work conducted by CDRH over the past 5 years that is being used to help build a national medical evaluation system. FDA provided a summary of some of the public-private partnerships that have been established to help develop the core program. The examples included the launch of MDEpiNet, establishment of the methodology center at Harvard that will provide methodological leadership, and the science and infrastructure center at Cornell. FDA provided an overview of the Predictable And Sustainable Implementation Of National (PASSION) registry launched in 2014, which will also pilot capabilities of conducting faster, more efficient randomized controlled trials (RCT) using registry infrastructure.

Stakeholders presented on the focused topic.

Two stakeholder groups presented their views on the benefits of developing a national evaluation system for devices and the regulatory use of clinical experience information, or "real-world" evidence. The stakeholders emphasized several potential benefits of developing this system: 1) more efficient enrollment leading to shorter and quicker trials at reduced trial costs; 2) easier patient follow-up; 3) more efficient collection of postmarket data; 4) better information on device use in diverse populations that often are not studied during the premarket phase, but may be candidates for device use after approval; and 5) harmonization with other national and international data sources. The stakeholder discussed one case study of the TASTE trial which used the infrastructure of a cardiovascular registry in Sweden to conduct an RCT at much lower cost per patient and faster enrollment than in classic RCTs. The stakeholder referenced another case study of labeling expansion for transcatheter aortic valve replacement (TAVR) in which the ACC/STS registry was used for postmarket evaluation in lieu of a new study. Another benefit is that the registries would support continuous evaluation of the device both before and after approval of the device. The stakeholders emphasized that several investments are needed to establish this system. These include development of best practices for the use of real world evidence throughout the product life cycle, enhancements to registries, and establishing a support system. The stakeholders expressed their support for using MDUFA IV funds to support postmarket surveillance and the development of policies for the TPLC approach.

One stakeholder presented comments to the record expressing support for Industry's inclusion of patient-centered endpoints in their development programs, and for the role that CDRH is taking in fostering the use of patient preference information in the medical device review and approval process. The stakeholder also expressed concern about

CDRH staffing levels to handle review of the increasing amounts of Patient Reported Outcome (PRO) data. The stakeholder also encouraged CDRH to identify disease areas where PRO development work can be advanced by consortia or other parties.

The next patient and consumer stakeholder meeting is scheduled for Monday, January 11, 2016.

End 10:51am