Stakeholder Meeting on MDUFA IV Reauthorization
October 26, 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 of the ongoing MDUFA IV negotiations, plan for future stakeholder meetings, and obtain stakeholders' views on the focus topics of: *getting expertise for specific patient populations, efforts to improve data transparency on demographic data, and inclusion of subpopulations in clinical studies.*

Participants

FDA

Malcolm Bertoni	Office of the Commissioner (OC)
Marc Caden	Office of Chief Counsel (OCC)
Sonja Fulmer	Center for Devices and Radiological Health (CDRH)
Louise Howe	OCC
Heather Howell	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

Cynthia Bens	Alliance for Aging Research
Paul Brown	National Center for Health Research
Victoria Burack	Consumers Union
Ryne Carney	Alliance for Aging Research
Shannon Curtis	American Medical Association
Diane Dorman	dDConsulting
Mark Fleury	American Cancer Society Cancer Action Network
Eric Gascho	National Health Council
Lisa Goldstein	American College of Cardiology
Marisol Goss	AAOS
Maureen Japha	FasterCures
Bennie Johnson	JDRF

Andrea Lowe	Society for Women's Health Research
Paul Melmeyer	National Organization for Rare Disorders
Ben Moscovitch	Pew Charitable Trusts
Christina Silcox	National Center for Health Research
Brian Smith	Research!America
Jessica Tyson	Avalere Health

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:

FDA noted that these stakeholder meetings are a very important part of the reauthorization process. These meetings allow FDA to hear the ideas and the feedback stakeholders have on the medical device user fee program and negotiation process.

The last negotiation meeting with industry was held on October 1, 2015. The meeting included a presentation of data requested by industry, presentation of FDA's information systems for premarket review, and discussion of the independent assessment and additional financial analysis. The minutes of that meeting are posted on the FDA web site. FDA and industry agreed to present final proposals during the next negotiation meeting in November 2015. The goal is to reach an agreement that can go into administration clearance by Spring of 2016, and conduct the public process for review of draft recommendations by Fall of 2016, so that the final recommendations can be delivered to Congress no later than January 15, 2017, as required by law.

FDA presented the list of potential future topics identified during the breakout sessions from the last meeting, and stakeholders identified the top three topics to be discussed at future patient/consumer stakeholder meetings:

The topics nominated as the top three priorities for discussion at future stakeholder meetings were:

- Patient engagement and science of patient input:
    - Additional discussion on incorporating patient perspectives in FDA reviews: Discuss FDA's and industry's resource constraints that limit the use of scientific data on patient preference (PP) and patient reported outcomes (PRO), the limitations of getting devices to market using such information, ways in which industry is incorporating and seeking such information (i.e., the role of this information in the development process) and how FDA uses that information.
    - Increasing the use and utility of patient registries
    - Patient input on appropriate endpoints

    - Update on FDASIA Section 1137 implementation

- Use of evidence from various sources for postmarket and premarket purposes
    - Additional information on unique device identifier and other patient safety efforts
    - More efficient collection and use of data from various sources, including device and patient registries
    - Striking the right balance between pre-market and postmarket evidence collection and pre-post shift

- Cross-center coordination related to areas such as combination products, companion diagnostics, etc., including areas for efficiencies, and consideration of how to ensure adequate user fee funding.

For the focus topic, FDA presented progress and ongoing efforts related to getting expertise for specific patient populations, efforts to improve data transparency, and inclusion of subpopulations in clinical trials.

FDA highlighted the importance of demographic subgroup data, and stated that FDA has a variety of statutory, regulatory and policy-related tools at its disposal that provide a framework for guiding medical product sponsors and FDA review teams in the collection, analyses and communication of data on diverse subgroup populations. FDA discussed section 907 of the Food and Drug Safety and Innovation Act, which directed the agency to look at specific demographic data in terms of inclusion in clinical trials, analyses for safety and effectiveness, and public availability of resulting information on performance of medical products in demographic subgroups. As directed under Section 907, FDA issued a Report in 2013 which included results of a retrospective review of 72 medical product applications, and determined that, in general, medical product developers/manufacturers and FDA staff are complying with relevant requirements and guidance. Nevertheless, areas for improvement were noted, and these findings were used to inform and guide development of the FDASIA Section 907-Action Plan, issued in 2014. In the action plan, FDA committed to 27 action items focused on three priority areas: 1) quality of demographic data, 2) participation of demographic subgroups in clinical trials, and 3) transparency to the public regarding demographic subgroup data. FDA provided an overview of progress on the Action Plan commitments, overseen by an agency-wide steering committee. CDRH in particular has incorporated recommendations from final guidance on sex and gender subgroup analysis into review templates, and is drafting guidance with recommendations on evaluation and reporting of age, race, and ethnicity data in medical device clinical studies. There will be a public meeting in February 2016 to gain insight on how the agency is doing and what FDA should do to move forward.

CDRH Chief Medical Officer for Pediatrics and Special Populations was introduced, and other related activities were briefly described, including draft guidance issued in 2015 on leveraging clinical data for extrapolation to pediatric uses of medical devices.

Stakeholder perspective on and questions about the program:

Stakeholders agreed that they are satisfied with the topics discussed and the reports on progress of the negotiations.

For the focus topic, they expressed their belief that the proportion of women, minorities and elderly patients in industry-sponsored clinical trials is not consistent with the prevalence of the disease in the underlying population, and that study enrollment criteria may result in some groups, including people with multiple conditions, being excluded from studies. Other stakeholders expressed concern about underpowered analyses as well as the need to understand performance of devices including through future iterations where the devices that are available are different from those initially studied.

The stakeholders stressed the importance of expanding the use of registries and other means of collecting data on device use once a device is broadly available on the U.S. market, which may also be a source of data on device use in diverse patients, including those with multiple conditions. The stakeholders continued to suggest consideration of user fees to cover the cost of post market surveillance, and underscored the importance of ensuring efficient and effective postmarket data collection and use in light of efforts to shift data collection in certain situations from premarket. The stakeholders expressed concerns with resources (user fees) being available to maintain current programs as well as for completing outstanding action items. The stakeholders encouraged the agency to take a total product lifecycle approach as pre- and post-market cannot be dissected from each other. The stakeholders stressed the importance of FDA having the funds to hire, train, and retain employees that are experts in the field to help evaluate the clinical trials and therapies. The stakeholders stated they were in support of LDTs being subject to FDA regulatory oversight.

Meeting End Time: 11:00 am