Stakeholder Meeting on PDUFA VI Reauthorization
November 16, 2015, 1:00 PM – 3:00 PM
FDA White Oak Campus, Silver Spring, MD

Purpose

To continue discussions of the current status of the human drug and biologic review programs in the context of PDUFA reauthorization. Topics for discussion were based on stakeholder perspectives shared at the July 15, 2015 public meeting and docket submissions.

Participants

FDA

Bob Ball	CDER
Steve Berman	CDER
Patrick Frey	CDER
Hector Izurieta	CBER
Andy Kish	CDER
Theresa Mullin	CDER
Mary Parks	CDER
Graham Thompson	CDER
Terry Toigo	CDER

Registered Stakeholders

Cynthia Bens	Alliance for Aging Research
Ron Bartek	Friedreich's Ataxia (FARA)
Paul Brown	National Center for Health Research
Ryne Carney	Alliance for Aging Research
Allison Durham	FaegreBD Consulting
Eric Gascho	National Health Council
Steve Gibson	The ALS Association
Rob Goldsmith	Cancer Support Network
Lisa Goldstan	American College of Cardiology
Amanda Grimm	Cystic Fibrosis Foundation
Lori Hoffman	Sarcoma Foundation of America
Anna Hyde	Arthritis Foundation
Bennie Johnson	Juvenile Diabetes Research Foundation
Maureen Japha	FasterCures
Ethan Jorgensen-Earp	American Academy of Pediatrics
Carol Kennedy	EveryLife Foundation of Rare Diseases
Ian Kremer	Leaders Engaged on Alzheimer's Disease
Bea Long	Environmental Protection Agency (EPA)
Andrea Lowe	Society for Women's Health Research
Amanda Pezalla	American Academy of Dermatology Association
Andrew Sperling	National Alliance on Mental Illness

Joseph Stewart	Health and Medicine Counsel of Washington
Jennifer Tripp	Muscular Dystrophy Association
Ernest Voyard	The Leukemia & Lymphoma Society, Office of Public Policy
Patrick Wildman	The ALS Association
John Wylam	National MS Society

Meeting Start Time: 1:00 PM

The meeting on November 16 focused on providing stakeholders an overview and update on postmarket drug safety activities, which had been identified as a top topic of interest to the stakeholders in the previous meeting in September.

Drug Safety and PDUFA

FDA began its presentation by providing a brief historical overview of the evolution of drug safety activities in the PDUFA era. PDUFA V (2012) focused on enhancements and modernizations to the drug safety systems, particularly with respect to Risk Evaluation and Mitigation Strategies (REMS) authorized under the Food and Drug Administration Amendments Act of 2007 (FDAAA), Sentinel, and pharmacovigilance processes. Commitments in these areas included public meetings, guidances, reports, tool development, and enhancement projects. FDA provided updates on progress made on these commitments and its plans for continued modernization of the US drug safety system, including: adoption of new scientific approaches; improving the utility of the tools for the detection, evaluation, prevention, and mitigation of adverse events; and enhancing collaboration between pre-market and post-market review staff.

Post-marketing Drug Safety Monitoring

FDA provided an overview of four of its core post-market safety areas: pharmacovigilance, pharmacoepidemiology, pharmaceutical risk management, and medication error prevention. FDA provided a detailed introduction to its Sentinel Initiative, begun in 2008 under FDAAA, to establish a postmarket risk identification and analysis system to link and analyze safety data from multiple sources. The future plans for Sentinel were described, including the move from the Mini-Sentinel pilot activities to the sustained active surveillance system and the development of enhanced methodological approaches.

Case Study: A Claims-based Big Data CMS Study of the High Dose Influenza Vaccine

FDA presented a case study on the use of the Centers for Medicare and Medicaid Services (CMS) database to examine data on the use of marketed medical products. The case study described the use of data from the CMS database to examine the use of high-dose influenza vaccine in elderly patients, following the accelerated licensure of the vaccine based upon surrogate endpoint trial data. The study also provided FDA experience and analytical methods that may be applicable for the study of other regulated products.

Question and Answer and Open Discussion

The Stakeholder discussion session began with technical questions regarding special populations and use of registries in postmarket regulatory activities. The use of user fee funding for observational studies of marketed medical products was discussed, as were plans for additional studies based on CMS data. In response to a stakeholder inquiry about the use of ex-US data in postmarket surveillance, FDA described its work on global regulatory harmonization and, when appropriate, the use of non-US datasets. The session concluded with a discussion of potential biases in observational studies and methods for controlling them.

Plan for Next Meeting
The Stakeholder Meeting on PDUFA VI Reauthorization is scheduled for December 17.

Meeting End Time: 3:00 PM