4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2013-N-1658]

Characterizing and Communicating Uncertainty in the Assessment of Benefits and Risks in Drug Regulatory Decision-Making; Public Workshop; Request for Comments

AGENCY: Food and Drug Administration, HHS

ACTION: Notice of Public Workshop; request for public comments.

SUMMARY: The Food and Drug Administration (FDA or the Agency) is announcing the following workshop convened by the Institute of Medicine (IOM): "Characterizing and Communicating Uncertainty in the Assessment of Benefits and Risks in Drug Regulatory Decision-Making." The purpose of the workshop is twofold: To explore potential approaches to addressing and communicating uncertainty and to identify key considerations on developing, evaluating, and incorporating potential approaches for addressing uncertainty into the assessment of benefits and risks in the human drug review process. The format of the meeting consists of a series of presentations on topics related to uncertainty in the assessment of benefits and risks, followed by a discussion on those topics with invited panelists and audience members. This workshop satisfies an FDA commitment that is part of the fifth authorization of the Prescription Drug User Fee Act (PDUFA V).

DATES: The public workshop will be held on February 12, 2014, from 8:30 a.m. to 4:30 p.m. and February 13, 2014, from 8:30 a.m. to 3:30 p.m. Registration to attend the public workshop must be received by January 31, 2014. See the SUPPLEMENTARY INFORMATION section

for information on how to register for the workshop. Submit either electronic or written comments by March 14, 2014.

ADDRESSES: The public workshop will be held at FDA's White Oak Campus, 10903 New Hampshire Ave., Building 31 Conference Center, rm. 1503A, Silver Spring, MD 20993-0002. Entrance for public workshop attendees (non-FDA employees) is through Building 1 where routine security check procedures will be performed. For parking and security information, please refer to http://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm.

Submit electronic comments to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. Identify all comments with the docket number found in brackets in the heading of this document.

FOR FURTHER INFORMATION CONTACT: Sara Eggers, Food and Drug Administration, Center for Drug Evaluation and Research, 10903 New Hampshire Ave., Bldg. 51, rm. 1166, Silver Spring, MD 20993-0002, 301-796-4904, FAX: 301-847-8443, email: sara.eggers@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

On July 9, 2012, the President signed into law the Food and Drug Administration Safety and Innovation Act (FDASIA) (Public Law 112-144). Title I of FDASIA reauthorizes PDUFA and provides FDA with the user fee resources necessary to maintain an efficient review process for human drug and biological products. The reauthorization of PDUFA includes performance

goals and procedures for the Agency that represents FDA's commitments during fiscal years 2013-2017. These commitments are fully described in the document entitled "PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2013 through 2017" (PDUFA Goals Letter), available on FDA's Web site at http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM270412.pdf.

Section X of the PDUFA Goals Letter, entitled "Enhancing Benefit-Risk Assessment in Regulatory Decision-Making," includes development of a plan to further develop and implement a structured approach to benefit-risk assessment in the human drug review process. As part of this enhancement, FDA committed to holding two public workshops on benefit-risk considerations from the regulator's perspective that will begin by the first quarter of fiscal year 2014. The public workshop announced in this notice will fulfill the first of the two workshop commitments.

As part of its commitment, FDA has published the "Structured Approach to Benefit-Risk Assessment in Drug Regulatory Decision-Making: Draft PDUFA V Implementation Plan," available on FDA's Web site at http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM329758.pdf. In this plan, FDA identified as an area of further development the exploration of structured approaches to evaluate and communicate the uncertainty in the assessment of benefits and risks. FDA's human drug regulatory decisions are informed by an extensive body of evidence on the safety and efficacy of a drug product. In many cases, this evidence is subject to uncertainty arising from many sources. One example is the uncertainty in the degree to which premarket clinical trial data translates to the postmarket setting after the drug is approved and used in a

much wider patient population. Another example is uncertainty about a potential safety signal that emerges in the postmarket setting, where the basis for the finding comes from multiple evidence sources of varying levels of rigor. Drawing conclusions in the face of uncertainty can be a complex and challenging task. Furthermore, being explicit about the impact of uncertainty on decision-making is an important part of communicating regulatory decisions.

II. Purpose and Scope of the Workshop

This 2-day public workshop is being convened by IOM. The public workshop will explore more systematic and structured approaches to evaluate and communicate: (1) the sources of uncertainty in the assessment of benefits and risks and (2) their implications on human drug regulatory decisions. Specifically, the workshop will explore potential analytical and communication approaches to addressing and communicating uncertainty and identify key considerations on developing, evaluating, and incorporating potential approaches for addressing uncertainty into the assessment of benefits and risks in the human drug review process. This public workshop will consider the entire drug development life cycle, including premarket drug review and postmarket safety surveillance. The format of the meeting consists of a series of presentations on topics related to uncertainty in the assessment of benefits and risks, followed by a discussion on those topics with invited panelists and audience members.

III. Attendance and Registration

FDA's Conference Center at the White Oak location is a Federal facility with security procedures and limited seating. Persons interested in attending the public workshop must register online by January 31, 2014. To register for the public workshop, please visit FDA's workshop Web site at http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm378861.htm. Early

registration is recommended. Registration is free and will be on a first-come, first-served basis. However, the number of participants from each organization may be limited based on space limitations. Registrants will receive confirmation once they have been accepted. Onsite registration on the day of the workshop will be based on space availability. If you need special accommodations because of disability, please contact Sara Eggers (see FOR FURTHER INFORMATION CONTACT) at least 7 days before the workshop. More information will be made available on FDA's workshop Web site at least 5 days before the workshop date.

A live Webcast of this workshop will be viewable on FDA's workshop Web site at http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm378861.htm on the day of the workshop. A video recording of the workshop will be available on FDA's workshop Web site approximately 1 week following the workshop. IOM will independently prepare a summary of the workshop, and the summary will be available on FDA's workshop Web site approximately 10 months following the workshop.

IV. Comments

Regardless of attendance at the public workshop, interested persons may submit either electronic comments or written comments to the Division of Dockets Management (see ADDRESSES). It is only necessary to send one set of comments. Identify comments with the docket number found in brackets in the heading of this document. To ensure consideration, submit comments by March 14, 2014. Received comments may be seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday, and will be posted to the docket at http://www.regulations.gov.

Dated: January 6, 2014.

Leslie Kux,

Assistant Commissioner for Policy.

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