4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2017-N-0001]

Developing a Framework for Regulatory Use of Real-World Evidence; Public Workshop

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop.

SUMMARY: The Food and Drug Administration (FDA, the Agency, or we) is announcing the following public workshop entitled "Developing a Framework for Regulatory Use of Real-World Evidence." Convened by the Duke-Robert J. Margolis, MD, Center for Health Policy at Duke University and supported by a cooperative agreement with FDA, the purpose of the public workshop is to bring the stakeholder community together to discuss a variety of topics related to the use of real-world data (RWD) and real-world evidence (RWE) in drug development and regulatory decision making. Topics will include an update on FDA's activities to address the use of RWE in regulatory decisions and the development of a framework for tackling challenges related to RWE's regulatory acceptability. In addition, panelists will discuss opportunities to improve data development activities, study designs, and analytical methods used to create robust RWE.

DATES: The public workshop will be held on September 13, 2017, from 9 a.m. to 4:30 p.m., Eastern Time.

ADDRESSES: The public workshop will be held at the Conference Center at 1777 F Street NW, Washington, DC 20006. For additional travel and hotel information, please refer to the

following website: https://healthpolicy.duke.edu/events/public-workshop-developing-framework-regulatory-use-real-world-evidence. There will also be a live webcast for those unable to attend the meeting in person (see Streaming Webcast of Public Workshop).

FOR FURTHER INFORMATION CONTACT: Kayla Garvin, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, rm. 6314, Silver Spring, MD 20993, (301) 796-7578, Kayla.Garvin@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

RWD (data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources) and RWE (clinical evidence regarding the usage and potential benefits or risks of a drug derived from analysis of RWD) are increasingly being used by multiple stakeholders within the health care system. Payers may rely on RWD and RWE to refine formularies or assist in coverage decisions. Physicians and professional societies can utilize RWE to further tailor clinical practice guidelines and decision-support tools. Medical product developers can use RWE to further develop a product's benefit-risk profile, monitor postmarket safety and adverse events, or generate additional hypotheses for continued clinical development.

The 21st Century Cures Act, section 3022 (Pub. L. 114-255), enacted on December 13, 2016, directed FDA to establish a program to evaluate the potential use of RWE. The framework of the program was to include information describing the sources of RWE, the gaps in data collection, standards and methods for collection and analysis, and the priority areas and challenges.

To date, RWD and RWE have been used in very specific regulatory contexts. Some treatments for rare diseases, for example, have utilized RWE as part of the historical controls used for clinical study and, ultimately, regulatory submission. Postmarket safety surveillance has also relied heavily on RWD-generating networks. As part of exploring the opportunities for enhanced use of these types of data and evidence in additional regulatory decision-making contexts, FDA is seeking input on the opportunities and challenges in using RWE to support the approval of a new indication for an already approved drug, and to help support or satisfy postapproval study requirements.

This public workshop is being held to engage external stakeholders in discussions around the current state of RWE development and potential challenge areas for using RWE in regulatory decisions beyond postmarket safety surveillance.

II. Topics for Discussion at the Public Workshop

During the course of the public workshop, speakers and participants will cover a range of issues related to the development of RWE and its applicability within specific regulatory decision-making contexts. This will include, but not be limited to, challenges related to RWD collection and quality, innovative methods for developing RWE from RWD, and promising areas for RWE pilot demonstrations.

III. Participating in the Public Workshop

Registration: To register for the public workshop, please visit the following website before September 12, 2017: https://healthpolicy.duke.edu/events/public-workshop-developing-framework-regulatory-use-real-world-evidence. There will be no onsite registration. Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone.

Registration is free and based on space availability, with priority given to early registrants. Early registration is recommended because seating is limited; therefore, FDA may limit the number of participants from each organization. A 1-hour lunch break is scheduled, but food will not be provided. There are multiple restaurants within walking distance of the Conference Center.

If you need special accommodations due to a disability, please contact Joanna Higgison at the Duke-Margolis Center for Health Policy, 908-432-4872, joanna.higgison@duke.edu, no later than September 6, 2017.

Streaming Webcast of the Public Workshop: This public workshop will also be webcast and archived video footage will be available at the Duke-Margolis website (https://healthpolicy.duke.edu/events/public-workshop-developing-framework-regulatory-use-real-world-evidence) following the workshop. Persons interested in viewing the live webcast must register online by September 12, 2017, at 5 p.m. Eastern Time (see Registration). Early registration is recommended because webcast connections are limited. Organizations are requested to register all participants, but to view using one connection per location whenever possible. Webcast participants will be sent technical system requirements in advance of the event. Prior to joining the streaming webcast of the public workshop, it is recommended that you review these technical system requirements.

FDA has verified the website addresses in this document, as of the date this document publishes in the Federal Register, but websites are subject to change over time.

Meeting Materials: All event materials will be provided to registered attendees via email prior to the workshop and publicly available at the Duke-Margolis website

(https://healthpolicy.duke.edu/events/public-workshop-developing-framework-regulatory-use-real-world-evidence).

Transcripts: Please be advised that transcripts will not be available.

Dated: July 25, 2017.

Leslie Kux,

Associate Commissioner for Policy.

[FR Doc. 2017-16021 Filed: 7/28/2017 8:45 am; Publication Date: 7/31/2017]