4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2013-N-0985]

Complex Issues in Developing Drug and Biological Products for Rare Diseases; Public Workshop; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop; request for comments.

The Food and Drug Administration (FDA) is announcing the following public workshop entitled "Complex Issues in Developing Drug and Biological Products for Rare Diseases." The purpose of the public workshop is twofold: To discuss complex issues in clinical trials for developing drug and biological products ("drugs") for rare diseases, including endpoint development and selection, use of surrogate endpoints and the accelerated approval pathway, clinical trial design, conduct and analysis, safety considerations, and dose selection; and to discuss ways to encourage and accelerate the development of new therapies for pediatric rare diseases. FDA is seeking input on these topics from academic, clinical, and treating communities; patients and advocacy groups; industry; and governmental agencies. Input from this public workshop will help develop a strategic plan to encourage and accelerate the development of new therapies for rare diseases.

Date and Time: The public workshop will be held on January 6, 2014, from 8 a.m. to 5 p.m. and on January 7, 2014, from 8 a.m. to 4:45 p.m.

Location: The public workshop will be held at FDA's White Oak Campus, 10903 New Hampshire Ave., Building 31 Conference Center, the Great Room (rm. 1503), Silver Spring, MD

20993-0002. Entrance for the public meeting participants (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.

Contact Person: Tomeka Arnett, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, rm. 6331, Silver Spring, MD 20993-0002, 301-796-2500, FAX: 301-847-3529, email: Tomeka.Arnett@fda.hhs.gov.

Registration: Registration is free and available on a first-come, first-served basis. Persons interested in attending the public workshop must register online by December 20, 2013. Early registration is recommended because facilities are limited and, therefore, FDA may limit the number of participants from each organization. If time and space permits, onsite registration on the day of the public workshop will be provided beginning at 7:30 a.m. Seating will be available on a first-come, first-served basis.

If you need special accommodations due to a disability, please contact Tomeka Arnett (see Contact Person) no later than 7 days in advance.

To register for the public workshop, please visit FDA's Drugs News & Events--Meetings, Conferences & Workshops calendar at http://www.fda.gov/Drugs/NewsEvents/ucm132703.htm. (Select this public workshop from the posted events list.) Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone number. Those without Internet access should contact Tomeka Arnett to register (see Contact Person). Registrants will receive confirmation after they have been accepted. You will be notified if you are on a waiting list.

Streaming Webcast of the Public Workshop: This public workshop will also be Webcast. Persons interested in viewing the Webcast may visit FDA's Drugs News & Events--Meetings, Conferences & Workshops calendar at http://www.fda.gov/Drugs/NewsEvents/ucm132703.htm. (Select this public workshop from the posted events list.) Select https://collaboration.fda.gov/drugbiord/ to view the Webcast. If you have never attended a Connect Pro event before, test your connection at https://collaboration.fda.gov/common/help/en/support/meeting_test.htm. (FDA has verified the Web site addresses in this document, but FDA is not responsible for any subsequent changes to the Web sites after this document publishes in the Federal Register.)

Comments: FDA is holding this public workshop to obtain information about complex issues in clinical trials for developing drugs for rare diseases and to discuss ways to encourage and accelerate the development of new therapies for pediatric rare diseases. In order to permit the widest possible opportunity to obtain public comment, FDA is soliciting either electronic or written comments on all aspects of the public workshop. The deadline for submitting comments regarding this public workshop is March 10, 2014.

Regardless of attendance at the public workshop, interested persons may submit either electronic comments regarding this document to http://www.regulations.gov or written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville MD 20852. 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. In addition, when responding to specific topics as outlined in section II, please identify the topic you are addressing. 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.

Transcripts: Please be advised that as soon as a transcript is available, it will be accessible at http://www.regulations.gov. A transcript will also be available in either hardcopy or on CD-ROM, after submission of a Freedom of Information request. Written requests are to be sent to the Division of Freedom of Information (ELEM-1029), Food and Drug Administration, 12420 Parklawn Dr., Rockville, MD 20857.

SUPPLEMENTARY INFORMATION:

I. Background

The Orphan Drug Act of 1983 (the Orphan Drug Act) (Public Law 97-414), as amended, defines a "rare disease or condition" to include those that affect less than 200,000 persons in the United States. This definition is codified in section 526(a)(2) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb(a)(2)). The Orphan Drug Act provides incentives to reduce the cost and increase the potential reward for developing products for small numbers of patients; however, it does not alter the statutory standards for marketing approval. To gain approval, all drugs must demonstrate substantial evidence of effectiveness, safety, and product quality for the treatment of the condition in the identified patient population. FDA acknowledges that certain aspects of drug development for rare diseases are challenging, and U.S. regulations allow for flexibility and scientific judgment in applying approval standards and in determining the kind and quantity of data required for a particular drug to meet the statutory standards.

This public workshop is being held in response to section 510--Pediatric rare diseases of the Food and Drug Administration Safety and Innovation Act (Public Law 122-144) (125 Stat. 1050), whereby FDA is required to hold at least one public meeting to discuss ways to encourage

and accelerate the development of new therapies for pediatric rare diseases. Additionally, as stated in section IX.E--Enhancing Regulatory Science and Expediting Drug Development, Advancing Development of Drugs for Rare Diseases of the Prescription Drug User Fee Act Reauthorization Performance Goals and Procedures Fiscal Years 2013 through 2017 (available at http://www.fda.gov/downloads/forindustry/userfees/prescriptiondruguserfee/ucm270412.pdf), FDA will conduct a public meeting to discuss complex issues in clinical trials for studying drugs for rare diseases.

This public workshop is being held in conjunction with FDA's Center for Devices and Radiological Health and Office of Orphan Products Development public workshop entitled "Complex Issues in Developing Medical Devices for Pediatric Patients Affected by Rare Diseases," which will be held on January 8, 2014, from 8 a.m. to 5 p.m., announced in a separate notice publishing elsewhere in this issue of the Federal Register.

II. Topics for Discussion at the Public Workshop

FDA is announcing a public workshop regarding complex issues in clinical trials for developing drugs for rare diseases and to discuss ways to encourage and accelerate the development of new therapies for pediatric rare diseases. The purpose of this public workshop is to seek broad input from rare disease experts and stakeholders, including industry; academic and clinical experts; patients and advocates and governmental agencies to address complex issues in rare disease product development.

Topics for discussion on day 1 include: (1) Complex issues for endpoints, including endpoint selection, use of surrogate endpoints and the accelerated approval pathway, clinical significance of primary endpoints, and development of patient-reported outcome instruments; (2) complex issues for trial design conduct and analysis; (3) development of translational and

regulatory science to support rare disease drug development; and (4) safety and dosing considerations, including safety exposures and assessment of dose selection.

Topics for discussion on day 2 include: (1) Collaborative research networks for pediatric rare diseases; (2) safety considerations for pediatric rare diseases; (3) pediatric rare cancers; and (4) development of gene therapies for rare pediatric disorders. Discussions will help develop a report that includes a strategic plan to encourage and accelerate the development of new therapies for pediatric rare diseases.

FDA encourages individuals, patients, advocates, industry, consumer groups, health care professionals, researchers and other interested persons to attend this public workshop.

Dated: September 17, 2013.

Leslie Kux,

Assistant Commissioner for Policy.

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