4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2018-N-3693]

Product Development in Hemophilia; 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 "Product Development in Hemophilia." The purpose of the public workshop is to discuss issues related to development and regulation of novel hemophilia products.

DATES: The public workshop will be held on December 6, 2018, from 8:30 a.m. to 4:30 p.m. See the SUPPLEMENTARY INFORMATION section for registration date and information.

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

Docket: For access to the docket to read background documents go to https://www.regulations.gov and insert the docket number, found in brackets in the heading of

this document, into the "Search" box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION CONTACT: Joan Ferlo Todd, Food and Drug Administration, Center for Drug Evaluation and Research, Office of Hematology and Oncology Products, 10903 New Hampshire Ave., Bldg. 22, Rm. 2139, Silver Spring, MD 20993-0002, 301-796-6079, Joan.Todd@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

Hemophilia is a bleeding disorder caused by deficiency of coagulation factor VIII (hemophilia A) or coagulation factor IX (hemophilia B). Hemophilia treatment strategies are intended to prevent or control bleeding and the attendant complications. Recently, hemophilia treatment strategies have led to the development of factor concentrates, recombinant DNA technology products, antibodies, and potential curative strategies such as gene therapy. These new emerging technologies raise new considerations about trial design, novel endpoints, patient-reported outcomes, and long-term safety collection.

This public workshop is intended to provide a platform for engaging in a discussion with experts in hemophilia treatment, patients, and caregivers. The purpose of this workshop is to advance further development of patient-experience and patient-reported outcomes for use in clinical trials, facilitate reliable and interpretable measurements of factor VIII/IX activity levels for gene therapy products, discuss the need for long-term safety assessments in gene therapy clinical trials, and discern when to enroll pediatric patients in gene therapy trials.

II. Topics for Discussion at the Public Workshop

The workshop will feature presentations and panel discussions on hemophilia product development. The presentations will include an overview of product development in hemophilia, and the regulatory challenges in the development of novel hemophilia therapies. Five sessions include presentations to frame panel discussions to cover the following topics:

1. Overview of product development in hemophilia;
2. Efficacy endpoints related to bleeding outcomes and considerations for factor activity as a surrogate endpoint;
3. Patient and caregiver perspectives on developing outcomes for clinical trials;
4. Discrepancies in the factor activity measurements by different assays observed in gene therapy trials and root causes for the discrepancies; and
5. Clinical trial design considerations for follow up on safety, efficacy, enrollment of pediatric patients in gene therapy trials, and the applicability of on-demand treatment as a control group in the evolving landscape of treatment options in hemophilia.

III. Participating in the Public Workshop

Registration: Persons interested in attending this public workshop must register online at https://fdaoce.formstack.com/forms/pdh120618 before 5 p.m. on December 3, 2018. 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. Registrants will receive confirmation when they have been accepted.

If you need special accommodations due to a disability, please contact Joan Ferlo Todd at Joan.Todd@fda.hhs.gov no later than 5 p.m., on November 21, 2018.

Streaming Webcast of the Public Workshop: This public workshop will also be web-streamed on the day of the workshop.

If you have never attended a webcast event before, test your connection at https://collaboration.fda.gov/common/help/en/support/meeting_test.htm. To get a quick overview of the Adobe webcast program, visit https://www.adobe.com/go/connectpro_overview. 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.

Transcripts: Please be advised that as soon as a transcript of the public workshop is available, it will be accessible at https://www.regulations.gov. It may be viewed at the Dockets Management Staff (see ADDRESSES). A link to the transcript will be available on the internet at https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm620602.htm.

Dated: October 29, 2018.

Leslie Kux,

Associate Commissioner for Policy.

[FR Doc. 2018-23947 Filed: 11/1/2018 8:45 am; Publication Date: 11/2/2018]