4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2014-N-0851]

Public Meeting on Patient-Focused Drug Development for Hemophilia A, Hemophilia B, von Willebrand Disease, and Other Heritable Bleeding Disorders

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public meeting; request for comments.

The Food and Drug Administration (FDA) is announcing a public meeting and an opportunity for public comment on Patient-Focused Drug Development for Hemophilia A, Hemophilia B, von Willebrand Disease, and other heritable bleeding disorders such as other factor deficiencies (including I, V, VII, X, XI) and platelet disorders. Patient-Focused Drug Development is an FDA performance commitment under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V). The public meeting is intended to provide FDA with patients' perspectives on the impact on daily life of Hemophilia A, Hemophilia B, von Willebrand Disease, and other heritable bleeding disorders. FDA also is seeking patients' perspectives on the available therapies for these disorders.

Dates and Time: The public meeting will be held on September 22, 2014, from 9 a.m. to 5 p.m. Registration to attend the meeting must be received by September 12, 2014. Registration from those individuals interested in presenting comments as part of the panel discussions must be received by August 22, 2014 (see the SUPPLEMENTARY INFORMATION for instructions).

Location: The meeting will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Bldg. 31 Conference Center, the Great Room (rm. 1503 B and C), Silver

Spring, MD 20993. Entrance for public meeting participants (non-FDA employees) is through Building 1, where routine security checks will be performed. For more information on parking and security procedures, please refer to http://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm.

Submit either electronic or written comments by November 28, 2014. 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. All comments should be identified with the docket number found in brackets in the heading of this document.

FDA will post the agenda approximately 5 days before the workshop at: http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/WorkshopsMeetingsConferences/ucm401758.htm.

Contact Person: Henry Allen, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, rm.1125, Silver Spring, MD 20993, 240-402-8001, FAX: 301-595-1243, email: PatientFocused_CBER@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background on Patient-Focused Drug Development

FDA has selected Hemophilia A, Hemophilia B, von Willebrand Disease, and other heritable bleeding disorders as the focus of a public meeting under the Patient-Focused Drug Development initiative. This initiative involves obtaining a better understanding of patients' perspectives on the challenges posed by these disorders, and the impact of therapies for these disorders. The Patient-Focused Drug Development initiative is being conducted to fulfill FDA

performance commitments that are part of the PDUFA reauthorization under Title I of the Food and Drug Safety and Innovation Act (Public Law 112-144). The full set of performance commitments is available on the FDA Web site at http://www.fda.gov/downloads/forindustry/userfees/prescriptiondruguserfee/ucm270412.pdf.

FDA has committed to obtaining the patient perspective on 20 disease areas during the course of PDUFA V. For each disease area, the Agency will conduct a public meeting to discuss the disease and its impact on patients' daily lives, the types of treatment benefits that matter most to patients, and patients' perspectives on the adequacy of the available therapies. These meetings will include participation of FDA review divisions, the relevant patient communities, and other interested stakeholders.

On April 11, 2013, FDA published a notice in the Federal Register (78 FR 21613) that announced the disease areas for meetings in fiscal years (FY) 2013-2015, the first 3 years of the 5-year PDUFA V timeframe. The Agency used several criteria outlined in the April 11, 2013, notice to develop the list of disease areas. FDA obtained public comment on the Agency's proposed criteria and potential disease areas through a public docket and a public meeting that was convened on October 25, 2012. In selecting the set of disease areas, FDA carefully considered the public comments received and the perspectives of review divisions at FDA. By the end of FY 2015, FDA will initiate a second public process for determining the disease areas for meetings in FY 2016-2017. More information, including the list of disease areas and a general schedule of meetings, is posted on FDA's Web site at http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm326192.htm.

II. Public Meeting Information

A. Purpose and Scope of the Meeting

The purpose of this Patient-Focused Drug Development meeting is to obtain input on the symptoms and other impacts that matter most to patients with Hemophilia A, Hemophilia B, von Willebrand Disease, and other heritable bleeding disorders. FDA also intends to seek patients' perspectives on current approaches to treating these disorders. FDA expects that this information will come directly from patients, caregivers, and patient advocates.

Heritable bleeding disorders are a diverse group of diseases and some involve lifelong defects in the clotting mechanism of the blood. The most frequently occurring of these disorders include Hemophilia A, Hemophilia B, and von Willebrand Disease. Less frequent yet also serious heritable bleeding disorders include Factor VII deficiency, Factor XIII deficiency, α2-antiplasmin deficiency and platelet disorders such as Gray platelet syndrome. Symptoms of heritable bleeding disorders include frequent nose bleed; prolonged and heavy menstrual bleeding; prolonged bleeding from cuts, trauma, dental extractions, and surgical procedures as well as bleeding into internal organs, muscles, and joints. Intracranial hemorrhage is a particularly serious and life-threatening manifestation. Specific treatment recommendations are determined by the type and severity of the disorder; but in general, therapies such as factor replacement, platelet transfusion, fresh frozen plasma, and cryoprecipitate are utilized.

The questions that will be asked of patients and patient stakeholders at the meeting are provided in this document. For each topic, a brief patient panel discussion will begin the dialogue. This will be followed by a facilitated discussion inviting comments from other patient and patient stakeholder participants. In addition to input generated through this public meeting, FDA is interested in receiving patient input addressing these questions through electronic or

written comments, which can be submitted to the Division of Dockets Management (see Location). For context, please indicate if you are commenting as a patient with a heritable bleeding disorder or on behalf of a child or loved one.

Topic 1: The effects of your bleeding disorder that matter most to you

- Of all of the symptoms that you experience because of your condition, which one to three symptoms (bleeding or non-bleeding) have the most significant impact on your life? (Examples may include joint damage/pain, infections, prolonged and heavy bleeding with menstruation, fatigue, etc.)
- Are there specific activities that are important to you, but that you cannot do at all, or as well as you would like, because of your condition? Please describe, using specific examples. (Examples may include participating in physical activities, attending work/school, and family/social activities, etc.)
- How have your condition and its symptoms changed over time?
- What worries you most about your condition?

Topic 2: Perspectives on current approaches to treatment

- What are you currently doing to treat your condition or its symptoms? (Examples may include blood transfusions, replacement therapies, over-the-counter products, and/or other therapies).
    - How well do these treatments work for you?
    - What are the most significant disadvantages or complications of your current treatments, and how do they affect your daily life?
    - How has your treatment changed over time and why?
    - What aspects of your condition are not improved by your current treatment regimen?

- What treatment has had the most positive impact on your life?

- If you could create your ideal treatment, what would it do for you (i.e., what specific things would you look for in an ideal treatment)?
- If you had the opportunity to consider participating in a clinical trial studying experimental treatments, what things would you consider when deciding whether or not to participate?

B. Attendance and/or Participation in the Meeting

If you wish to attend this meeting, visit https://www.eventbrite.com/e/patient-focused-public-meeting-on-heritable-bleeding-disorders-registration-11996980291. Please register by September 12, 2014. Those who are unable to attend the meeting in person can register to view a live Webcast of the meeting. You will be asked to indicate in your registration if you plan to attend in person or via the Webcast. Your registration will also contain your complete contact information, including name, title, affiliation, address, email address, and phone number. Seating will be limited, so early registration is recommended. Registration is free and will be on a first-come, first-served basis. However, FDA may limit the number of participants from each organization based on space limitations. Registrants will receive confirmation once they have been accepted. Onsite registration on the day of the meeting will be based on space availability. If you need special accommodations because of disability, please contact Henry Allen (see Contact Person) at least 7 days before the meeting.

Patients and patient stakeholders who are interested in presenting comments as part of the initial panel discussions should register by August 22, 2014. You will be asked to indicate in your registration which topic(s) you wish to address. You will be asked to send a brief summary of responses to the topic questions to PatientFocused_CBER@fda.hhs.gov. Panelists will be

notified of their selection soon after August 22, 2014. FDA will try to accommodate all patients and patient advocate participants who wish to speak, either through the panel discussion or audience participation; however, the duration of comments may be limited by time constraints.

Comments: Interested members of the public, including those who attend the meeting in person or via the Webcast, are invited to provide electronic or written responses to any or all of the questions pertaining to topics 1 and 2 to the Division of Dockets Management (see Location). Comments may be submitted until November 28, 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.

Transcripts: Please be advised that as soon as a transcript is available, it will be accessible at http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/WorkshopsMeetingsConferences/ucm401761.htm and at http://www.regulations.gov. It may be viewed at the Division of Dockets Management (see Location). 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., Element Bldg., Rockville, MD 20857.

Dated: July 2, 2014.

Leslie Kux,

Assistant Commissioner for Policy.

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