4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2017-N-3068]

Patient-Focused Drug Development for Hereditary Angioedema; Public Meeting; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public meeting; request for comments.

SUMMARY: The Food and Drug Administration (FDA, the Agency, or we) is announcing a public meeting and an opportunity for public comment on "Patient-Focused Drug Development for Hereditary Angioedema." Patient-Focused Drug Development is part of FDA's performance commitment under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V). The public meeting is intended to allow FDA to obtain patients' perspectives on the impact of hereditary angioedema (HAE) on daily life. FDA also is seeking patients' views on treatment approaches for HAE.

DATES: The public meeting will be held on September 25, 2017, from 9 a.m. to 3 p.m. Registration to attend must be received by August 10, 2017. Submit either electronic or written comments on the public meeting by November 20, 2017. See the SUPPLEMENTARY INFORMATION section for registration date and information.

ADDRESSES: The public meeting will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Bldg. 31 Conference Center, the Great Room (rm. 1503), Silver Spring, MD 20993. 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 https://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm.

You may submit comments as follows. Please note that late, untimely filed comments will not be considered. Electronic comments must be submitted on or before November 20, 2017. The https://www.regulations.gov electronic filing system will accept comments until midnight Eastern Time at the end of November 20, 2017. Comments received by mail/hand delivery/courier (for written/paper submissions) will be considered timely if they are postmarked or the delivery service acceptance receipt is on or before that date.

Electronic Submissions

Submit electronic comments in the following way:

- Federal eRulemaking Portal: https://www.regulations.gov. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to https://www.regulations.gov will be posted to the docket unchanged. Since your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on https://www.regulations.gov.

- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see "Written/Paper Submissions" and "Instructions").

Written/Paper Submissions

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852.
- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in "Instructions."

Instructions: All submissions received must include the Docket No. FDA-2017-N-3068 for "Patient-Focused Drug Development for Hereditary Angioedema." Received comments, those filed in a timely manner (see ADDRESSES), will be placed in the docket and, except for those submitted as "Confidential Submissions," publicly viewable at https://www.regulations.gov or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states "THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION." The Agency will review this copy, including the claimed confidential information, in its

consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on https://www.regulations.gov. Submit both copies to the Dockets Management Staff. If you do not wish your name and contact information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as "confidential." Any information marked as "confidential" will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA's posting of comments to public dockets, see 80 FR 56469, September 18, 2015, or access the information at: https://www.gpo.gov/fdsys/pkg/FR-2015-09-18/pdf/2015-23389.pdf.

Docket: For access to the docket to read background documents or the electronic and written/paper comments received, 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.

FDA will post the agenda approximately 5 days before the meeting at http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/WorkshopsMeetingsConferences/ucm542319.htm.

FOR FURTHER INFORMATION CONTACT: Barbara Kass, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, rm. 1125, Silver Spring, MD 20993, 240-402-6887; or Loni Warren Henderson, Center for Biologics

Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, rm. 1118, Silver Spring, MD 20993, 240-402-8180, PatientFocused_CBER@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background on Patient-Focused Drug Development

FDA has selected HAE 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 HAE and the impact of current therapies for this condition. 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 Administration Safety and Innovation Act (Pub. L. 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 committed to obtaining the patient perspective on 20 disease areas during the course of PDUFA V. For each disease area, the Agency is conducting 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), announcing the disease areas for meetings in fiscal years (FYs) 2013-2015, the first 3 years of the 5-year PDUFA V time frame. The Agency used several criteria outlined in that 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. FDA initiated a second public process for determining the disease areas for FY 2016-2017 and published a notice in the *Federal Register* on July 2, 2015 (80 FR 38216), announcing the selection of eight disease areas. More information, including the list of disease areas and a general schedule of meetings, is posted at https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm.

II. Purpose and Scope of the Meeting

As part of the Patient-Focused Drug Development, FDA will obtain input on the symptoms and other aspects of the disease that matter most to patients with HAE. FDA also intends to seek patients' perspectives on current approaches to treating HAE. FDA expects that this information will come directly from patients, caregivers, and patient advocates.

HAE is a rare genetic disorder that affects less than 200,000 individuals in the United States. It is associated with episodic recurrent attacks of swelling of the body caused by abnormalities in a protein called C1-Esterase Inhibitor. Most cases occur because there is either not enough of the protein or because the protein does not work normally to help prevent swelling of the body.

In individuals with HAE, the swelling attacks may involve various areas of the body, including the gastrointestinal tract, arms, legs, face, or throat and larynx (voice box). Symptoms of this condition often begin during childhood but may also appear in adulthood. The swelling episodes are usually self-limited; may or may not be associated with any triggering factors; and in severe cases involving the larynx, may be life-threatening. If not recognized early and left untreated, swelling of the larynx, called laryngeal edema, may acutely restrict airflow to the

lungs and could result in death. Gastrointestinal tract swellings are often associated with nausea, vomiting, and abdominal pain, which can be severe and require hospitalization. Several FDA-approved therapies affecting different biological mechanisms are available to treat or prevent acute attacks of HAE.

The questions that will be asked of patients and patient representatives at the meeting are listed in this section and organized by topic. The two main topics for discussion are: (1) Symptoms and impact on activities of daily life that matter most to patients; and (2) perspectives on current approaches to treatment. For each topic, a brief patient/caregiver panel discussion will begin the dialogue. This will be followed by a facilitated discussion, inviting comments from other patient and caregiver 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 Dockets Management Staff (see ADDRESSES). For context, please indicate if you are commenting as a patient with HAE or on behalf of a child or loved one.

Topic 1: Disease Symptoms and Daily Impacts That Matter Most to Patients

(1) Of all of the symptoms that you experience because of your condition, which one of these symptoms has the most significant impact on your life? Examples may include nausea, vomiting, abdominal pain, swelling of extremities, facial swelling, tongue swelling, hoarseness or loss of voice, shortness of breath, and difficulty urinating.

(2) Are there specific activities that are important to you 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; and attending work or school and family or social activities, during or between attacks.

(3) How have your condition and its symptoms changed over time?

(4) What worries you most about your condition?

Topic 2: Patients' Perspectives on Current Approaches to Treatment

(1) What are you currently doing to treat your condition and its symptoms?

- What, if anything, are you doing to prevent acute HAE attacks? Examples may include treatments with prescription medicines; over-the-counter products; and other therapies, including non-drug therapies.
- What, if anything, do you self-administer for acute HAE attacks?
- If you give yourself medication for acute HAE attacks, which types of attacks, with respect to body location(s), are you comfortable treating yourself?
- What treatment has your health professional used for your acute HAE attacks? Examples may include prescription medicines; over-the-counter products; and other therapies, including non-drug therapies.

(2) How well do these treatments work for you?

(3) What are the most significant disadvantages or complications of your current treatments, and how do they affect your daily life?

(4) How has your treatment regimen changed over time and why?

(5) What aspects of your condition are not improved by your current treatment regimen?

(6) What treatment has had the most positive impact on your quality of life?

(7) Short of a complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?

(8) 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?

III. Meeting Attendance and Participation

Registration: If you wish to attend this meeting, visit https://www.eventbrite.com/e/patient-focused-drug-development-for-hereditary-angioedema-public-meeting-tickets-32300298061. Persons interested in attending this public meeting must register by August 10, 2017. If you are unable to attend the meeting in person, you 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. 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 once they have been accepted. Onsite registration on the day of the meeting will be based on space availability. If you need special accommodations due to a disability, please contact Barbara Kass or Loni Warren Henderson (see FOR FURTHER INFORMATION CONTACT) no later than September 18, 2017.

Requests for Oral Presentations: Patients and patient representatives who are interested in presenting comments as part of the initial panel discussions will be asked to indicate in their registration which topic(s) they wish to address. These patients and patient representatives also must send to PatientFocused_CBER@fda.hhs.gov a brief summary of responses to the topic questions by [INSERT DATE 14 DAYS AFTER DATE OF PUBLICATION IN THE FEDERAL REGISTER]. Panelists will be notified of their selection approximately 7 days before the public meeting. We will try to accommodate all patients and patient representatives

who wish to speak, either through the panel discussion or audience participation; however, the duration of comments may be limited by time constraints.

Transcripts: Please be advised that, as soon as a transcript of the public meeting 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 also be available on the Internet at http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/WorkshopsMeetingsConferences/ucm542320.htm.

Dated: July 13, 2017.

Anna K. Abram,

Deputy Commissioner for Policy, Planning, Legislation, and Analysis.

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