4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2015-N-1798]

Patient-Focused Drug Development for Alpha-1 Antitrypsin Deficiency; Public Meeting

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public meeting; request for comments.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing a public meeting and an opportunity for public comment on Patient-Focused Drug Development for Alpha-1 Antitrypsin Deficiency (AATD). 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 AATD. FDA also is seeking patients' perspectives on the available therapies for this disorder.

DATES: The public meeting will be held on September 29, 2015, from 9 a.m. to 3:30 p.m. Registration to attend the meeting must be received by September 15, 2015. Registration from those individuals interested in presenting comments as part of the panel discussions should be received by July 31, 2015. See the SUPPLEMENTARY INFORMATION section for instructions on how to register for the meeting. Submit either electronic or written comments by November 30, 2015.

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

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.

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, FAX: 301-595-1243, email: PatientFocused_CBER@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background on Patient-Focused Drug Development

FDA has selected AATD 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 AATD 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 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. FDA has initiated a second public process for determining the disease areas for meetings in FY 2016-2017 and published a notice in the Federal Register on October 8, 2014 (79 FR 60857). 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. 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 AATD. FDA also intends to seek

patients' perspectives on current approaches to treating this disorder. FDA expects that this information will come directly from patients, caregivers, and patient advocates.

Individuals with AATD have low serum levels of Alpha-1-Antitrypsin (AAT, also known as Alpha-1 proteinase inhibitor (A1-PI)) and increased risks of developing a form of chronic obstructive lung disease called emphysema and, less frequently, liver disease. Some AATD patients with emphysema have symptoms of asthma. There are different genetic forms of the disease, but even among people with the same genetic form and similar levels of AAT in their blood, there is tremendous diversity in clinical severity. A substantial percentage of individuals with severe AATD never develop symptomatic lung disease during their lifetimes. Others may develop the first signs and symptoms of lung disease between the ages of approximately 25 and 50 years, or older. Affected individuals often develop emphysema, which is a lung disease caused by damage to the small air sacs in the lung. Progression of emphysema in AATD may lead to respiratory failure, a need for lung transplantation, and eventually death. The only specific medication approved for raising the blood levels of AAT in severe AATD patients with emphysema is weekly intravenous treatment with A1-PI (Human) purified from human blood plasma.

Severe AATD patients may also develop liver disease as infants, during childhood, or as adults. There is wide variation in the severity of liver disease among affected patients. Currently, no specific therapy for AATD-related liver disease is available other than liver transplantation, so the focus in these patients is on the prevention and management of other types of liver damage.

The questions that will be asked of patients and patient caregivers at the meeting are provided in this document. The meeting will be divided into two main topics: (1) The effects of

Alpha-1 Antitrypsin Deficiency that matter most to you, and (2) perspectives on current approaches to treatment. 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 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 Division of Dockets Management (see ADDRESSES). For context, please indicate if you are commenting as a patient with AATD or on behalf of a child or loved one.

Topic 1: The effects of Alpha-1 Antitrypsin Deficiency that matter most to you.

- Of all of the symptoms that you experience because of your condition, which one to three symptoms have the most significant impact on your life? (Examples may include:
  (a) For lung disease: shortness of breath during specific activities or at rest, chronic cough, wheezing, weight loss, exacerbations of particular symptoms;
  (b) For liver disease: abdominal pain, loss of appetite, height and weight concerns.)
- 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.)
- 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:

(a) For lung disease: inhaled bronchodilators, inhaled corticosteroids, intravenous augmentation therapy with A1-PI (Human) on a regular or intermittent basis;

(b) For liver disease: ursodiol.)

- 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?

III. Attendance and Registration

If you wish to attend this meeting, visit https://www.eventbrite.com/e/public-meeting-on-patient-focused-drug-development-for-alpha-1-antitrypsin-deficiency-tickets-15617092143. If you do not have access to the Internet, you may mail or fax your registration information (including name, title, affiliation, address, email address, telephone, and fax numbers) to Barbara Kass (see FOR FURTHER INFORMATION CONTACT) by September 15, 2015. There is no registration fee for the public meeting. Early registration is recommended because seating is limited. FDA may limit the number of participants from each organization based on space limitations. Registrants will receive confirmation once they have been accepted. Registration on

the day of the public meeting will be provided on a space available basis beginning at 8 a.m. Those who are unable to attend the meeting in person can register to view a live Web cast of the meeting. You will be asked to indicate in your registration if you plan to attend in person or via the Web cast.

If you need special accommodations because of disability, please contact Barbara Kass at least 7 days in advance. FDA will post the agenda approximately 5 days before the meeting at http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/MeetingsMeetingsConferences/ucm435242.htm.

IV. Comments

Patients and patient caregivers who are interested in presenting comments as part of the panel discussions should register by July 31, 2015. You will be asked to indicate in your registration which topic(s) you wish to address and to send a brief summary of responses to the topic questions to PatientFocused_CBER@fda.hhs.gov by July 31, 2015. Panelists will be notified of their selection soon after August 28, 2015. FDA will try to accommodate all patients and patient caregivers who wish to speak, either through the panel discussion or audience participation; however, the duration of comments may be limited by time constraints.

Regardless of attendance at the public meeting, interested persons may submit either electronic or written responses to any or all of the questions pertaining to topics 1 and 2 to the Division of Dockets Management (see ADDRESSES). 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. To ensure consideration, submit comments by November 30, 2015. 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.

V. Transcripts

Please be advised that as soon as a transcript is available, it will be accessible at http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/WorkshopsMeetingsConferences/ucm435242.htm and at http://www.regulations.gov. It may also be viewed at the Division of Dockets Management (see ADDRESSES). 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: May 26, 2015.

Leslie Kux,

Associate Commissioner for Policy.

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