4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2012-N-0967]

Prescription Drug User Fee Act Patient-Focused Drug Development; Public Meeting and Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public meeting; request for comments.

SUMMARY: The Food and Drug Administration (FDA) is announcing a public meeting and an opportunity for public comment related to FDA's patient-focused drug development initiative. This initiative is being conducted to fulfill FDA performance commitments made as part of the fifth authorization of the Prescription Drug User Fee Act (PDUFA V). This effort provides for a more systematic approach under PDUFA V for obtaining patient perspective on the disease severity and the currently available treatments for a set of disease areas. FDA is publishing a preliminary list of nominated disease areas for the patient-focused drug development initiative and the criteria used for nomination. The public is invited to comment on this preliminary list through a public docket and at a public meeting where FDA will provide an overview of the patient-focused drug development initiative with discussion of the nominated disease areas.

DATES: Submit either electronic or written comments by November 1, 2012. The public meeting will be held on October 25, 2012, from 9 a.m. to 12:30 p.m. Registration to attend the meeting must be received by October 18, 2012. See the SUPPLEMENTARY INFORMATION section for information on how to register for the meeting.

ADDRESSES: The 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 20993-0002. 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.

Submit electronic comments to 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:

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SUPPLEMENTARY INFORMATION:

I. Background

On July 9, 2012, the President signed into law the Food and Drug Administration Safety and Innovation Act of 2012 (FDASIA). Title I of FDASIA reauthorizes the Prescription Drug User Fee Act (PDUFA) that provides FDA with the necessary user fee resources to maintain an efficient review process for human drug and biologic products. The reauthorization of PDUFA includes performance goals and procedures that represent FDA's commitments during fiscal years (FY) 2013-2017. These commitments are referred to in section 101 of FDASIA and are available on the FDA Web site at http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM270412.pdf.

Section X of these commitments relates to enhancing benefit-risk assessment in regulatory decisionmaking. A key part of regulatory decisionmaking is establishing the context in which the particular decision is made. In drug regulation, this context includes a thorough understanding of the severity of the treated condition and the adequacy of the existing treatment options. Patients who live with a disease have a direct stake in the outcome of the review process and are in a unique position to contribute to weighing benefit-risk considerations that can occur throughout the medical product development process. Though several programs exist to facilitate patient representation, there are currently few venues in which the patient perspective is discussed outside of a specific product's marketing application review. The human drug and

biologic review process could benefit from a more systematic and expansive approach to obtaining input from patients who experience a particular disease or condition.

FDA is committed to obtaining input from patients and, as set out in the commitment letter, will conduct public meetings to consider 20 different disease areas over the 5-year authorization of the program. For each disease area, FDA will conduct a public meeting to discuss such topics as the impact of the disease on patients, the spectrum of severity for those who have the disease, the measures of benefit that matter most to patients, and the adequacy of the existing treatment options for patients. These meetings will include participation of FDA review divisions, the relevant patient advocacy community, and other interested stakeholders. FDA seeks public comment on the set of disease areas that will be discussed at these meetings throughout PDUFA V. A preliminary list of possible disease areas and the criteria used to identify these disease areas are published in this notice for public comment.

FDA recognizes that there is significant interest in patient-focused drug development within the patient community. To ensure that patient stakeholders have an opportunity to contribute as this initiative moves forward in PDUFA V, FDA also is convening an additional series of patient consultation meetings with patient stakeholders to discuss key process questions for patient-focused drug development. These consultation meetings will be separate from the disease-specific meetings that are part of FDA's commitments in PDUFA V. FDA has published a separate notice elsewhere in this issue of the Federal Register requesting that patient stakeholders notify FDA if they intend to participate in the patient consultation meetings.

II. Disease Area Nomination

FDA is nominating the following disease areas as potential candidates for the focus of one of the 20 future public meetings and invites public comment on this preliminary list. In your

comments, please identify the disease areas that you consider to be of greatest priority and explain the rationale for your recommendation.

- Pulmonary arterial hypertension
- Heart failure
- Primary glomerular diseases
- Narcolepsy
- Huntington's Disease
- Depression
- Autism
- Peripheral neuropathy
- Fibromyalgia
- Obesity
- Nocturia
- Chronic fatigue syndrome
- Irritable bowel syndrome
- Inflammatory bowel disease
- Alopecia areata
- Diabetic ulcers
- Female sexual dysfunction
- Interstitial cystitis/painful bladder syndrome
- Fracture healing
- Diabetic foot infections
- Hepatitis C

- HIV
- Patients who have experienced an organ transplant
- Sickle cell disease
- Chronic graft versus host disease
- Amyloidosis
- Aplastic anemia
- Melanoma
- Lung cancer
- Cancer and young patients
- Cancer treatment in pregnancy
- Cancer and sexual dysfunction
- Cancer and depression
- Clotting disorders (e.g., hemophilia A (factor VIII deficiency) and von Willebrand disease)
- Thrombotic disorders (e.g., antithrombin deficiency and protein C deficiency)
- Primary humoral immune deficiencies (e.g., common variable immune deficiency)
- Neurologic disorders treated with immune globulins (e.g., chronic inflammatory demyelinating polyneuropathy)
- Hereditary angioedema
- Alpha-1 antitrypsin deficiency

FDA is also interested in public comment on disease areas that are not represented on this preliminary list. The Agency used several criteria to develop the preliminary list of potential

disease areas. FDA requests that when proposing additional disease areas for consideration, please describe how you applied the identified criteria in making recommendations for additional disease areas to consider.

FDA also welcomes public comment on the criteria for disease area selection. These criteria include the following:

- Disease areas that are chronic, symptomatic, or affect functioning and activities of daily living;
- Disease areas that reflect a range of severity;
- Disease areas for which aspects of the disease are not formally captured in clinical trials;
- Disease areas that have a severe impact on identifiable subpopulations (such as children or the elderly);
- Disease areas that represent a broad range in terms of size of the affected population; or
- Disease areas for which there are currently no therapies or very few therapies, or the available therapies do not directly affect how a patient feels, functions, or survives.

FDA will consider the public comments received at the public meeting and through the docket and post the set of disease areas for FY 2013-2015 on the FDA Web site. By the end of FY 2015, FDA will initiate a public process for determining the list of disease areas for FY 2016-2017.

III. Public Meeting

FDA is holding a public meeting that will begin FDA's patient-focused drug development initiative in PDUFA V. The purpose of this meeting will be to obtain public

comment on the preliminary list of potential disease areas and the criteria used to develop the list. In addition, recognizing that there are many more disease areas than can be addressed in the 20 planned FDA meetings for PDUFA V, FDA will also discuss strategies that have already been pursued by patient and other public stakeholder collaborations outside of FDA to address the types of questions being considered under the PDUFA patient-focused drug development effort, to review lessons learned and identify a roadmap that could be used by patient-focused private collaborations going forward.

If you wish to attend this meeting, please register by email to PatientFocused@fda.hhs.gov by October 18, 2012. Your email should contain 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 Andrea Tan (see FOR FURTHER INFORMATION CONTACT) at least 7 days before the meeting.

Dated: September 14, 2012.

Leslie Kux,

Assistant Commissioner for Policy.

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