



4164-01-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; Announcement of Disease Areas for Meetings Conducted in Fiscal Years 2016-2017

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of Availability.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the selection of disease areas to be addressed during fiscal years (FYs) 2016-2017 of its Patient-Focused Drug Development Initiative. This initiative is being conducted to fulfill FDA's performance commitments under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V). This effort provides a more systematic approach under PDUFA V for obtaining the patients' perspective on disease severity and currently available treatments for a set of disease areas. FDA selected these disease areas based on a careful consideration of the public comments received after publication of a preliminary list of disease areas in the Federal Register on October 8, 2014.

ADDRESSES: The general schedule of FYs 2016-2017 Patient-Focused Drug Development meetings, along with materials from past meetings (such as transcripts and webcast recordings) from past meetings, can be found at the Web site for Patient-Focused Drug Development, <http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm326192.htm>. Individual comments may be viewed at <http://www.regulations.gov/#!documentDetail;D=FDA-2012-N->

[0967-0595](tel:0967-0595) or by visiting the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852, between 9 a.m. and 4 p.m., Monday through Friday.

FOR FURTHER INFORMATION CONTACT: Graham Thompson, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, rm. 1146, Silver Spring, MD 20993, 301-796-5003, FAX: 301-847-8443, email:

PatientFocused@fda.hhs.gov, or Stephen Ripley, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, rm. 7301, Silver Spring, MD 20993, 240-402-7911.

SUPPLEMENTARY INFORMATION:

I. Background

On July 9, 2012, the President signed into law the Food and Drug Administration Safety and Innovation Act (FDASIA) (Pub. L. 112-144). Title I of FDASIA reauthorizes the Prescription Drug User Fee Act (PDUFA), which 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 FYs 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 assessments in regulatory decision making. A key part of regulatory decision making is establishing the context in which the particular decision is made. For purposes of drug marketing approval, this includes

an understanding of the severity of the treated condition and the adequacy of the available therapies. Patients who live with a disease have a direct stake in the outcome of FDA's decisions and are in a unique position to contribute to the Agency's understanding of their disease.

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

II. Disease Area Selection

On October 8, 2014, FDA published a Federal Register notice (79 FR 60857) that requested public comment on potential disease areas to be addressed in FYs 2016-2017. In that notice, based on several criteria listed, FDA identified 16 disease areas as potential candidates for remaining public meetings and invited public comment on the preliminary list and on disease areas that were not listed.

Following publication of the notice, almost 2,700 comments addressing over 50 disease areas were submitted by patients, patient advocates and advocacy groups, caregivers, healthcare providers, professional societies, scientific and academic experts, pharmaceutical companies, and others. The majority of comments received were submitted by individual patients. The comments focused generally on nominating individual disease areas or groups of disease areas to be addressed and on providing general suggestions for the Patient-Focused Drug Development Initiative. The comments received also discussed the impact of these nominated diseases on the patients' daily lives, the symptoms that were most concerning to patients, and the nature of (or

lack of) specific treatments for these diseases. The majority of comments received concerned lewy body dementia, frontotemporal lobar degeneration, and neuropathies. Other disease areas, such as hereditary angioedema, dystonia, temporomandibular disorders, lupus, alopecia areata, chronic lymphocytic leukemia, trigeminal neuralgia, and arachnoiditis, also received a significant number of comments.

In selecting the disease areas of focus for the Patient-Focused Drug Development Initiative of FYs 2016-2017, FDA carefully considered the valuable public comments received, the perspectives of reviewing divisions at FDA, and the following selection criteria, which were published in the October 8, 2014, Federal Register notice:

- Disease areas that are chronic, symptomatic, or affect functioning and activities of daily living;
- Disease areas for which aspects of the disease are not formally captured in clinical trials;
- 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; and
- Disease areas that have a severe impact on identifiable subpopulations (such as children or the elderly).

FDA's selection also reflects disease areas from FDA review divisions that were not covered by the meetings held during FYs 2013-15. For its FYs 2016-2017 list of disease areas, FDA has added a broad range of diseases based upon disease severity (less severe to more severe) and upon the size of the affected population (rare diseases to more prevalent diseases).

FDA has identified the following diseases to be the focus of meetings scheduled in FYs 2016-2017:

- Alopecia areata
- Autism
- Hereditary angioedema
- Non-tuberculous mycobacterial infections
- Patients who have received an organ transplant
- Psoriasis
- Neuropathic pain associated with peripheral neuropathy
- Sarcopenia

A schedule of the meetings planned can be found at the FDA Patient-Focused Drug Development Web site, which is described in section III of this notice. The Agency recognizes that there are many more disease areas than can be addressed in the planned FDA meetings under the formal PDUFA V commitment, and FDA will seek other opportunities to gather public input on disease areas not addressed through this Patient-Focused Drug Development Initiative. FDA encourages stakeholders to identify and organize patient-focused collaborations to generate public input on other disease areas using the process established through this Patient-Focused Drug Development Initiative as a model. Information on additional opportunities for gathering patient input can be found on the Patient-Focused Drug Development Web site.

III. Patient-Focused Drug Development Web Site

FDA's Web site on Patient-Focused Drug Development is available online at <http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm326192.htm>. This Web site contains the general schedule of upcoming meetings for FYs 2016-2017, information on how

stakeholders can prepare for these upcoming meetings, and information on how stakeholders may leverage the Patient-Focused Drug Development Initiative to generate input on disease areas not addressed through the Patient-Focused Drug Development PDUFA V commitment. The Web site will be updated as new information becomes available.

Dated: June 26, 2015.

Leslie Kux,

Associate Commissioner for Policy.

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