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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; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Request for comments.

SUMMARY: The Food and Drug Administration (FDA) is announcing 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 the patient perspective on disease severity and currently available treatments for a set of disease areas. FDA is publishing a preliminary list of nominated disease areas for consideration in patient-focused drug development meetings during fiscal years (FYs) 2016-2017. The public is invited to comment on this preliminary list through a public docket.

DATES: Submit either electronic or written comments by December 5, 2014.

ADDRESSES: 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: Pujita Vaidya, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, rm. 1144,

Silver Spring, MD 20993, 301-796-7641, FAX: 301-796-0684, Pujita.Vaidya@fda.hhs.gov; or Christopher Joneckis, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, rm. 7316, Silver Spring, MD 20993-0002, 240-402-8083, Christopher.Joneckis@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

On July 9, 2012, the President signed into law the Food and Drug Administration Safety and Innovation Act (FDASIA) of 2012. 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 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 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 the patient perspective on 20 different disease areas during the course of PDUFA V (FY 2013-2017). For each disease area, the Agency is conducting 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.

On April 11, 2013, FDA published a Federal Register notice (78 FR 21613) announcing the disease areas for meetings in FYs 2013-2015, the first 3 years of PDUFA V. In selecting the set of disease areas, FDA carefully considered the public comments received and the perspectives of review divisions at FDA. FDA is initiating a second public process for determining the disease areas for FYs 2016-2017. A preliminary list of possible disease areas and the criteria used to identify these disease areas are published in this document for public comment. FDA will consider the public comments received through the public docket and publish the set of disease areas for FYs 2016-2017 in a Federal Register notice.

II. Disease Area Nomination

FDA is nominating the following disease areas as potential candidates for the focus of the remaining public meetings in FYs 2016- 2017 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.

- Achondroplasia

- Alopecia areata
- Autism
- Autoimmune disorders treated with immune globulins
- Depression
- Diabetic foot infection
- Hereditary angiodema
- Melanoma, specifically unresectable loco-regional disease
- Neurologic disorders treated with immune globulins
- Nontuberculous mycobacterial infections
- Ovarian cancer
- Patients who have received an organ transplant
- Primary humoral immune deficiencies
- Pruritis
- Sarcopenia
- Thrombotic disorders

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. In the series of disease area meetings, the final disease set should reflect a range of diseases with respect to disease severity (less severe to more severe) and represent a broad range in terms of the size of the affected population (e.g., including more prevalent diseases as well as rare diseases). 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. The criteria include the following:

- 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).

III. Comments

Interested persons may submit either electronic comments regarding this document to http://www.regulations.gov or written comments 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. 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.

Dated: October 2, 2014.

Leslie Kux,

Assistant Commissioner for Policy.

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