

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2013-N-0815]

Narcolepsy Public Meeting on Patient-Focused Drug Development

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 on Patient-Focused Drug Development for narcolepsy. Patient-Focused Drug Development is part of FDA's performance commitments in 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 narcolepsy on daily life as well as the available therapies for narcolepsy.

DATES: The public meeting will be held on September 24, 2013, from 1 p.m. to 5 p.m.

Registration to attend the meeting must be received by September 13, 2013. See the

SUPPLEMENTARY INFORMATION section for information on how to register for the meeting. Submit electronic or written comments by November 25, 2013.

ADDRESSES: The meeting will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Building 31 Conference Center, Section A of the Great Room (rm. 1503), Silver Spring, MD 20993. Entrance for the public meeting participants is through Building 1, where routine security check procedures will be performed. For more information on parking and security procedures, 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.

FDA will post the agenda approximately 5 days before the meeting at:

<http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm359018.htm>.

FOR FURTHER INFORMATION CONTACT: Pujita Vaidya, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, rm. 1170, Silver Spring, MD 20993, 301-796-0684, FAX: 301-847-8443, email:

Pujita.Vaidya@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background on Patient-Focused Drug Development

FDA has selected narcolepsy to be the focus of a meeting under Patient-Focused Drug Development, an initiative that involves obtaining a better understanding of patients' perspectives on the severity of the disease and the available therapies for the condition. Patient-Focused Drug Development is being conducted to fulfill FDA's performance commitments made as part of the authorization of PDUFA under Title I of the Food and Drug Safety and Innovation Act (FDASIA) (Public Law 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 obtain the patient perspective in 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 benefit 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 community, and other interested stakeholders.

On April 11, 2013, FDA published a notice in the Federal Register (78 FR 22613) announcing the disease areas for meetings in fiscal years (FY) 2013-2015, the first 3 years of the 5-year PDUFA V timeframe. To develop the list of disease areas, the Agency used several criteria that were outlined in the April 11 notice. The Agency gathered public comment on these criteria and potential disease areas through a notice for public comment published in the Federal Register on September 24, 2012 (77 FR 55849), and through a public meeting held on October 25, 2012. In selecting the disease areas, FDA carefully considered the public comments received and the perspectives of its review divisions. By the end of FY 2015, FDA will initiate another public process for determining the disease areas for FY 2016-2017. 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. Public Meeting Information

A. Purpose and Scope of the Meeting

As part of Patient-Focused Drug Development, FDA will gather patient and patient stakeholder input on symptoms of narcolepsy that matter most to patients and on current approaches to treating narcolepsy. Narcolepsy is a chronic disorder of the central nervous system caused by the brain's inability to control sleep-wake cycles and is characterized by excessive daytime sleepiness, cataplexy, hallucination, and disturbed nocturnal sleep. Although

there is no cure for narcolepsy, medications and lifestyle modifications can help patients manage their symptoms. FDA is interested in obtaining a better understanding of patients' perspectives on the severity of the disease and assessments of available therapies.

The questions that will be asked of patients and patient stakeholders at the meeting are listed in this section, organized by topic. For each topic, a brief patient panel discussion will begin the dialogue, followed by a facilitated discussion inviting comments from other patient and patient stakeholder participants. In addition to input generated through this public meeting, FDA is interested in receiving patient input addressing these questions through the public docket (see ADDRESSES).

Topic 1: Disease symptoms and daily impacts that matter most to patients:

1. Of all 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 excessive daytime sleepiness, cataplexy, etc.)
2. Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your condition? (Examples of activities may include sleeping through the night, work and school performance, etc.)
3. How have your symptoms changed over time?
 - 3.1. Do your symptoms come and go? If so, do you know of anything that makes your symptoms better? Worse?

Topic 2: Patients' perspectives on current approaches to treating narcolepsy:

1. What are you currently doing to help treat your condition or its symptoms? (Examples may include FDA-approved medicines, over-the-counter products, and other therapies including non-drug therapies such as lifestyle modifications.)

- 1.1. What specific symptoms do your therapies address?
- 1.2. How has your treatment regimen changed over time, and why?
2. How well does your current treatment regimen treat the most significant symptoms of your disease?
 - 2.1. How well do these therapies improve your ability to do specific activities that are important to you in your daily life?
 - 2.2. How well have these therapies worked for you as your condition has changed over time?
3. What are the most significant downsides to your current therapies, and how do they affect your daily life? (Examples of downsides may include bothersome side effects, inconvenient dosing schedules, access issues, etc.)
4. Assuming there is no complete cure for your condition, what specific things would you look for in an ideal therapy for your condition?

B. Meeting Attendance and/or Participation

If you wish to attend this meeting, visit <http://patientfocusednarcolepsy.eventbrite.com>. Please register by September 13, 2013. Those who are unable to attend the meeting in person can register to view a live webcast of the meeting. You will be asked to indicate in your registration whether you plan to attend in person or via the webcast. Your registration should also contain your 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 Pujita Vaidya (see FOR FURTHER INFORMATION CONTACT) at least 7 days before the meeting.

Patients 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. They will also be asked to send a brief summary of responses to the topic questions to PatientFocused@fda.hhs.gov. Panelists will be notified of their selection soon after the close of registration on September 13, 2013. FDA will try to accommodate all patients and patient stakeholders who wish to speak, either through the panel discussion or audience participation; however, the duration of comments may be limited by time constraints.

Interested members of the public, including those who attend the meeting in person or through the webcast, are invited to provide electronic or written responses to the questions pertaining to Topics 1 and 2 to the public docket (see ADDRESSES). Comments may be submitted until November 25, 2013.

Dated: July 15, 2013.

Leslie Kux,

Assistant Commissioner for Policy.