4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2012-N-0962]

# Drug Development for Chronic Fatigue Syndrome and Myalgic Encephalomyelitis; Public Workshop

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop; request for comments.

The Food and Drug Administration (FDA), Center for Drug Evaluation and Research, is announcing a public workshop to discuss how best to facilitate and expedite the development of safe and effective drug therapies to treat signs and symptoms related to chronic fatigue syndrome (CFS) and myalgic encephalomyelitis (ME). FDA has determined that CFS and ME are serious conditions for which there are no approved drug treatments. On April 25, 2013, as part of FDA's Patient-Focused Drug Development initiative, patients will provide feedback on disease impact on quality of life and individual experience with current treatment regimens. On April 26, 2013, there will be discussions with academic and Government experts, patient advocates, patients, and clinicians on how to identify sound, quantitative outcome measures that can be used in clinical trials to determine whether disease symptoms improve with specific drug interventions.

Date and Time: The public workshop will be held on April 25, 2013, from 1 p.m. to 5 p.m., and on April 26, 2013, from 8:30 a.m. to 5 p.m.

Location: The public workshop will be held at the Bethesda Marriott, 5151 Pooks Hill Rd., Bethesda, MD 20814, 301-897-9400, Fax: 301-897-0192.

Contact Persons:

Mary Gross,

Center for Drug Evaluation and Research,

Food and Drug Administration,

10903 New Hampshire Ave.,

Silver Spring, MD 20993-0002,

301-796-3519,

Mary.Gross@fda.hhs.gov;

Randi Clark,

Center for Drug Evaluation and Research,

Food and Drug Administration,

10903 New Hampshire Ave., Silver Spring, MD 20993-0002,

301-796-4287,

Randi.Clark@fda.hhs.gov; or

Sara Eggers,

Center for Drug Evaluation and Research,

Food and Drug Administration,

10903 New Hampshire Ave., Silver Spring, MD 20993-0002,

301-796-4904,

Sara.Eggers@fda.hhs.gov.

Registration and Requests to Participate in Panel Discussions: If you wish to attend the public workshop or participate in a panel discussion, you must register by submitting an electronic or written request by 5 p.m. on April 8, 2013. Submit electronic requests to http://mecfsmeeting.eventbrite.com. Submit written requests to Mary Gross, Randi Clark, or

Sara Eggers (see Contact Persons). You must provide your name and business, organization, or personal affiliation as applies (e.g., industry, government, patient). Patients who are interested in presenting comments as part of the initial panel discussions may indicate which topic(s) they wish to address (see section II of this document).

The public workshop is free and seating will be on a first-come, first-served basis. We recommend that you register early because seating is limited. FDA may limit both the number of participants from individual organizations and the total number of attendees, based on space limitations. Registrants will receive confirmation once they have been accepted to attend the meeting. For those who cannot attend in person, a live Webcast of the meeting will be located at http://mecfsmeeting.eventbrite.com. For information about joining the meeting via Webcast, please go to http://www.fda.gov/Drugs/NewsEvents/ucm319188.htm.

FDA will post an agenda of the public workshop and other background material 5 days before the workshop at http://www.fda.gov/Drugs/NewsEvents/ucm319188.htm.

You may submit questions about the public workshop to ME-CFS-Meeting@fda.hhs.gov prior to the April 25 and 26 workshop dates.

If you need special accommodations because of a disability, contact Mary Gross, Randi Clark, or Sara Eggers (see Contact Persons) at least 7 days in advance.

Comments: Submit either electronic or written comments by April 8, 2013, to receive consideration. 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. 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. Electronic or written comments will be accepted after the meeting until August 2, 2013.

FDA will also hold an open public comment period on April 25 to give the public an opportunity to comment on topics that may not have been addressed in the discussion of topics 1 and 2 (see section II of this document). Workshop participants should register to participate in the open public comment period by April 8, 2013, and will be asked to provide a brief summary of their comments.

SUPPLEMENTARY INFORMATION

## I. Background

The Food and Drug Administration, Center for Drug Evaluation and Research, is announcing a scientific workshop to discuss how best to facilitate and expedite the development of safe and effective drug therapies to treat signs and symptoms related to CFS and ME. FDA has determined that CFS and ME are serious conditions for which there are no approved drug treatments. On April 25, 2013, patients will give feedback on disease impact on quality of life and their experiences with current treatment regimens. On April 26, 2013, there will be discussions with academic and Government experts, patient advocates, patients, and clinicians on how to identify sound, quantitative outcome measures to determine whether disease symptoms improve with specific interventions. For purposes of this workshop, the terms “CFS” and “ME” have been used interchangeably in describing the conditions. These terms are used as a frame of reference only. The terms are intended to be inclusive and make no judgment on the cause of different symptom complexes. Drug development focuses on quantitative measures of benefit (e.g., symptom improvement) in either the entire population or in a defined subset, not on the

name of the disease. In some cases, evaluating symptoms individually may be the optimal approach, while in others, evaluating a constellation of symptoms may be better.

## II. Purpose and Scope of the Public Workshop

FDA has selected CFS and ME to be the focus for a workshop under the Patient-Focused Drug Development initiative, an effort that involves obtaining a better understanding of patients' perspectives on the severity of the disease and assessment of currently available treatment options. Patient-Focused Drug Development is being conducted to fulfill FDA performance commitments made as part of the authorization of the Prescription Drug User Fee Act 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.

On Day 1 of the workshop (April 25, 2013) FDA will gather patients' perspectives on CFS and ME as part of the Patient-Focused Drug Development initiative. Day 1 will focus on two main topics: (1) Disease symptoms and daily impacts that matter most to patients; and (2) Patients' perspectives on current approaches to treating CFS and ME. Discussion questions for topics 1 and 2 are as follows:

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

1. What are the most significant symptoms that you experience resulting from your condition? (Examples may include prolonged exhaustion, confusion, muscle pain, heat or cold intolerance.)

2. What are the most negative impacts on your daily life that result from your condition and its symptoms? (Examples may include difficulty with specific activities, such as sleeping through the night.)

   a. How does the condition affect your daily life on the best days and worst days?

b. What changes have you had to make in your life because of your condition?

Topic 2: Patients' perspectives on current approaches to treating CFS and ME

1. What treatments are you currently using 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 activity limitations).

a. What specific symptoms do your treatments address?

b. 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?

a. Have these treatments improved your daily life (for example, improving your ability to do specific activities)? Please explain.

b. How well have these treatments worked for you as your condition has changed over time?

c. What are the most significant downsides of these treatments (for example, specific side effects)?

For each of these topics, a brief initial patient panel discussion will begin the dialogue, followed by a facilitated discussion inviting comments from other patient participants. FDA has not yet identified the panel participants. As part of the meeting registration, patients who are interested in presenting comments as part of the initial panel discussions may indicate which topic(s) they wish to address and will be asked to provide a brief summary of responses to the questions listed below. FDA will confirm with patients who have been identified to provide comments as part of the opening panel discussion in advance of the workshop.

FDA will try to accommodate all participants who wish to speak on Day 1, either through the panel discussions, audience participation, or the open public comment period; however, the duration of comments may be limited by time constraints. Those who are unable to attend the meeting in person, but who would like to provide their perspective on the discussion questions for topics 1 and 2 are invited to submit electronic or written comments to the Division of Docket Management (see Comments).

Day 2 of the workshop (April 26, 2013), will include a scientific discussion on how best to facilitate and expedite the development of safe and effective drug therapies for signs and symptoms related to CFS and ME. Presentations and panel discussions will include the following:

- lessons learned from previous studies;
- the role of drug repurposing;
- pathways to expediting drug therapies;
- appropriate clinical trial design in CFS and ME;
- outcome measures to assess efficacy; and
- potential valid endpoint measurements of symptom improvement

## III. Transcripts

Please be advised that a transcript of the workshop will be available for review at the Division of Dockets Management (see Comments) and on the Internet at http://www.regulations.gov. The 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: March 6, 2013.

Leslie Kux,

Assistant Commissioner for Policy.

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