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Drug Development for CFS and ME: Public Workshop



Patient-Focused Drug Development

April 25, 2013

Bethesda Marriott

5151 Pooks Hill Road,

Bethesda, MD 20814



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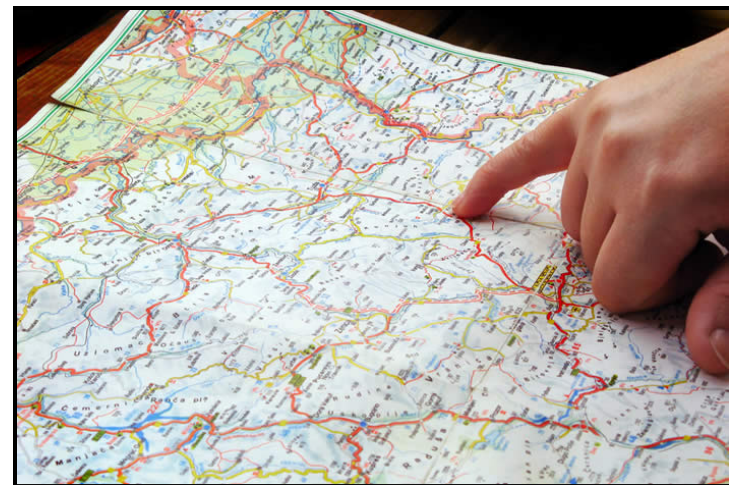
Welcome

RADM Sandra Kweder, MD

Deputy Director, Office of New Drugs
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

What to expect

- Clarity on high impact signs and symptoms
 - from patients
- Insight to clinical decision making
 - How physicians think about treatments
 - How patients respond to them
- What has been learned in clinical trials about how to study CFS/ME
- Drug development tools to facilitate sound and timely research and action
- We are building the map



Objectives

Listen and learn

Day 1

- To engage patients and patient representatives
- Most significant symptoms and negative impacts of disease
- Range of therapies



Day 2

- Examine common issues in drug development
- Consider tools – scientific and regulatory

Move forward!

Chronic Fatigue Syndrome

- Serious, complex, and debilitating disease
- Unknown etiology
- Characterized by profound fatigue >6 months duration; worsened by physical or mental activity
- Multiple body systems affected
- No diagnostic tests
- No approved therapies
- Lack of consensus on nomenclature and disease definition





Nomenclature

Chronic Fatigue Syndrome and
Myalgic Encephalomyelitis (CFS and ME)

Drug Development



Agenda

- Overview of Patient Focused Drug Development Initiative (*Theresa Mullin, PhD*)
- Introduction to the Discussion Format (*Sara Eggers, PhD*)
- Discussion Topic 1: Disease Symptoms/Impacts
- Discussion Topic 2: Treatments
- Open Public Comment Period
- Closing Remarks



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Thank You!



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Patient-Focused Drug Development

Theresa Mullin, Ph.D.

Director, Office of Planning and Informatics
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

April 25, 2013

Patient-Focused Drug Development Overview

- **FDA is developing a more systematic way of gathering patient perspective on their condition and available treatment options**
 - Patient perspective helps inform our understanding of the context for the assessment of benefit-risk and decision making for new drugs
 - This perspective could contribute more broadly to drug development efforts, for example, pointing to a need for new outcome measures in clinical trials
- **Patient-Focused Drug Development is part of FDA commitments under the fifth reauthorization of the Prescription Drug User Fee Act (PDUFA V)**
 - FDA will convene at least 20 meetings on specific disease areas over the next five years
 - FDA expects that patients, patient advocates, drug developers, and other interested parties will attend these meetings

Initiating the Process

- **In September 2012, FDA announced a preliminary set of diseases as potential meeting candidates**
 - Public input on these nominations was collected through an online docket and at a public meeting held in October 2012
 - Over 4,500 comments were submitted, which addressed over 90 disease areas
 - FDA carefully considered these public comments and the perspectives of our drug review divisions at FDA
- **The disease areas that will be the focus of meetings for fiscal years 2013-2015 have been announced in the Federal Register**
 - Another public process will be initiated in 2015 to determine the set for fiscal years 2016-2017

Considerations on Identifying Disease Areas

- **FDA sought a diverse set of disease areas that represent the range of diseases the Agency encounters in its regulatory decision-making**
- **We took into account the following overarching considerations:**
 - 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 or functions
 - Disease areas that reflect a range of severity, from diseases that are life-threatening to those that are mild and symptomatic
 - 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, including common conditions experienced by large numbers of patients and rare diseases that affect much smaller patient populations

Design Considerations

- **In planning the format and questions for each meeting, we will consider the unique characteristics of the disease context**
 - This context includes the current state of drug development and the specific needs of the patient population
 - Each meeting will focus a set of questions that aim to elicit patients' perspectives on the severity of their disease and on treatment approaches
 - Meeting formats will be tailored based on a general design to most effectively engage patients in dialogue
- **Patients and other stakeholders can contribute their perspectives in multiple ways**
 - Patients and patient representatives are invited to contribute to the meeting discussion
 - Anyone is invited to submit comments to the docket
- **A meeting report will be posted on our website, capturing the input we obtain in response to these key questions**
- **More information can be found on our website:**
<http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm326192.htm>



Overview of Discussion Format

Sara Eggers, PhD

Office of Planning and Analysis

Office of Planning and Informatics

Center for Drug Evaluation and Research

U.S. Food and Drug Administration

Discussion Topics

Topic 1: Most significant symptoms of CFS and ME, and their impact on daily life

- Significant symptoms that you experience resulting from your condition
- Negative impacts on your daily life— in particular, important activities that you cannot do at all, or as fully as you would like

Topic 2: Patient perspective on treating CFS and ME

- What are you currently doing to help treat your condition or its symptoms?
- How well do these treatments address your most significant symptoms?
- Downsides of treatment

Discussion Format – Topic 1 and 2

- **We will first hear from a panel of patients and representatives**
 - The purpose is to set a good foundation for our discussion
 - Panel members reflect a range of experiences with CFS and ME
- **We will then broaden the discussion to include other patients and patient representatives**
 - The purpose is to build on the experiences shared by the panel
 - The facilitator will ask follow up questions, inviting participants to raise hands to comment
- **Those participating by live webcast can add comments through the webcast comment box**
 - Although they will not be read or summarized today, they will be considered part of the public record

Discussion Ground Rules

- We encourage patients, caregivers and other patient representatives to contribute to the dialogue
- FDA staff is here to listen
- Our discussion will focus on understanding the common ground regarding the symptoms, impacts and treatment of CFS and ME
- Participant feedback on the meeting is important
- Respect for one another is paramount



Discussion Topic 1:

Disease Symptoms and Daily Impacts That Matter Most to Patients

Sara Eggers, PhD

Theresa Toigo, RPh, MBA

Associate Director for Drug Safety Operations
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

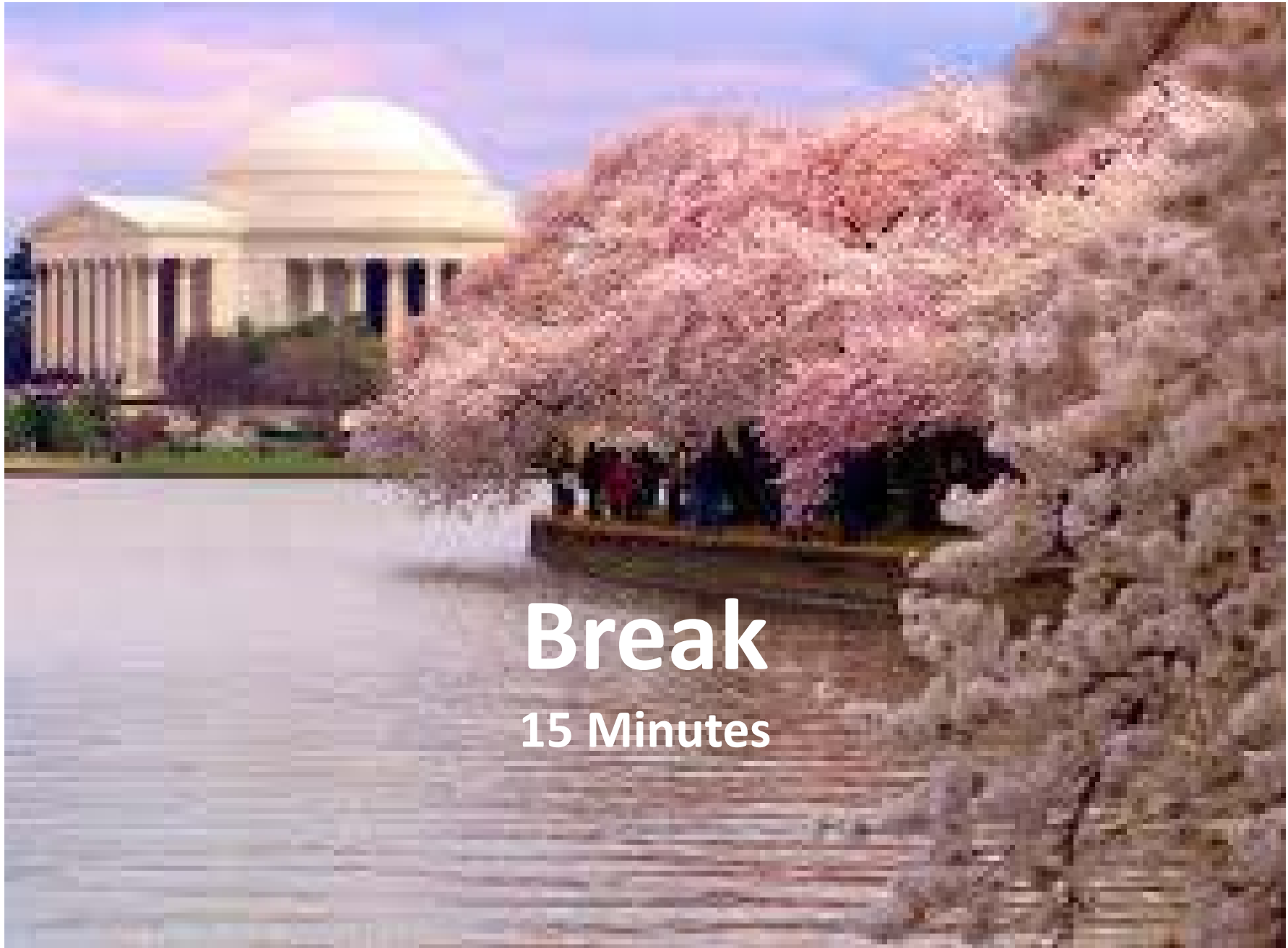


Topic 1 Panel Participants

- Jon Kaiser
- Joseph Landson
- Denise Lopez-Majano
- Kim McCleary
- Charlotte von Salis

Discussion Questions

- **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, etc.
- **What are the most negative impacts on your daily life that result from your condition and its symptoms?**
 - Examples: difficulty with specific activities, sleeping through the night, etc.
 - How does the condition affect your daily life on the best days and worst days?
 - What changes have you had to make in your life because of your condition?



CHERRY BLOSSOMS ON THE TIDAL BASIN



Discussion Topic 2:

Patients' Perspective on Treatment Approaches

Sara Eggers, PhD

Theresa Toigo, RPh, MBA

Topic 2 Panel Participants

- Mary Dimmock
- Tasha Kelemen
- Matina Nicholson
- Mary Schweitzer
- Amanda Simpson

Discussion Questions

- **What treatments are you currently using to help treat your condition or its symptoms?**
 - Consider prescription medicines, over-the-counter products and non-drug therapies such as activity limitations.
 - What specific symptoms do your treatments address?
 - How has your treatment regimen changed over time and why?
- **How well does your current treatment regimen treat the most significant symptoms of your disease?**
 - How well have these treatments improved your daily life, for example, improving your ability to do specific activities?
 - How well have these treatments worked for you as your condition has changed over time?
 - What are the most significant downsides of these treatments (for example, specific side effects)?



Open Public Comment Period

Open Public Comment Period Speakers

- Michael Walzer
- Anita Patton
- Courtney Alexander
- Steven Chilinski
- Judy Mikovits
- Derek Enlander
- Diane Lewis
- Thomas Equels
- Gisela Morales-Barreto
- David Strayer
- Dan Peterson
- Mary Silvey
- Eileen Holderman
- James Baraniuk
- Charles Lapp
- Steven Lempert
- Dwight Merriman
- Joan Grobstein
- Jeannette Burmeister



Closing Remarks

Theresa Mullin, PhD