MAYO CLINIC
College of Medicine

FDA Guidance on Patient Reported Outcomes:

Discussion, Dissemination, and Operationalization

February 23-25, 2006

Westfields Marriott
Chantilly, Virginia

Co-Sponsored by Mayo Clinic College of Medicine and the FDA's Center for Drug Evaluation and Research (CDER)

MAYO CLINIC
College of Medicine

FDA

For questions regarding participation contact:
Mayo School of Continuing Medical Education
Telephone: 800-323-2688 or 507-284-2509
Web: www.mayo.edu
FAX: 507-284-0532
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For general information and/or funding support of this meeting, please contact Dr. Jeff A. Sloan by phoning 507-284-9985, by faxing 507-266-2477, or by e-mailing jsloan@mayo.edu.

General Information

Course Description

In clinical research, patient reported outcomes (PROs) have been a focus of much discussion in recent years. The need for and manner of inclusion of PROs in clinical trials is a topic of great debate. Specific issues have arisen with respect to pharmaceutical industry sponsored trials, new drug applications, and labeling claims. The U.S. Food and Drug Administration (FDA) recently has constructed a draft guidance document for assessing PROs in clinical trials. The driving principle for this meeting is to create an open and inclusive discussion of the FDA guidance document. This open registration meeting, co-sponsored by Mayo Clinic College of Medicine and FDA Center for Drug Evaluation and Research (CDER), will be of interest to regulatory agency personnel, scientists, pharmaceutical industry personnel, clinicians and patient advocates.

Writing teams have been constructed to prepare written material covering five major themes: Conceptual Issues; PRO Instrument Selection; PRO Instrument Development Issues; Validation of PROs; and Analysis, Interpretation and Reporting Results Based on PROs.

The draft documents will be posted on the web for registered participants prior to the meeting. The draft manuscripts will form the basis of discussion at the meeting. Full and interactive input on the draft document will be encouraged from all participants at the open meeting. This is an opportunity for all stakeholders to have broad participation and constructive input into how PROs are incorporated into all clinical trials. Upon completion of the meeting, the writing teams will revise the draft documents to incorporate the discussions that will have taken place. These documents will be submitted to the FDA for consideration in preparing the final guidance document on this issue. This is a unique opportunity to get immediate and tangible feedback regarding PROs from the FDA. The FDA will also produce a manuscript in response to the material from the meeting.

The written documents from this meeting will be published in a peer-reviewed journal of high scientific merit. In brief, these documents will describe in detail that which is not possible in FDA guidance documents, which are concise by design. The publication will assist those in the medical product arena in operationalizing the material in the FDA guidance document in an efficient and effective manner. The commitment from the FDA to co-sponsor the meeting, be intrinsically involved in the planning process, and respond to the products of the meeting provide a vital added dimension and credibility to this effort.

Course Learning Objectives

Upon conclusion of this program, participants should be able to:

- Discuss the recent FDA draft guidance on PROs with all stakeholders.
- Describe the background, content, intent, and concerns surrounding the draft guidance.
- List the draft guidance implications.
- Delineate revisions needed in the draft guidance that represent the state of the science for using PROs as endpoints in clinical trials.
- Identify the value PROs add to information for clinicians and patients about the outcomes of treatment alternatives.

Attendance at this Mayo course does not indicate nor guarantee competence or proficiency in the performance of any procedures which may be discussed or taught in this course.

General Information

Credit

This activity has been planned and implemented in accordance with the Essential Areas and Policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint sponsorship of Mayo Clinic College of Medicine and FDA Center for Drug Evaluation and Research (CDER). Mayo Clinic College of Medicine is accredited by the ACCME to provide continuing medical education for physicians.

Mayo Clinic College of Medicine designates this educational activity for a maximum of 13.5 category 1 credits toward the American Medical Association (AMA) Physician's Recognition Award (PRA). Each physician should claim only those credits that he/she actually spent in the activity.

Non-US Licensed Physicians
The American Medical Association has determined that non-US licensed physicians who participate in this Continuing Medical Education activity are eligible for AMA PRA category 1 credit.

Other Health Care Professionals
A certificate of attendance will be provided to other health care professionals for requesting credits in accordance with state nursing boards, specialty societies, or other professional associations.

Educational Grants

This course is supported, in part, by educational grants from various companies, in accordance with ACCME Standards. Appropriate acknowledgment will be given to all supporters at the time of the meeting.

Participation in this meeting and membership on the writing teams are separate from and not related to support of the course.

Date and Location

The *"FDA Guidance on Patient Reported Outcomes: Discussion, Dissemination, and Operationalization"* Continuing Medical Education course will be held February 23-25, 2006. Course headquarters will be located in the Jeffersonian Ballroom in the Westfields Marriott, Chantilly, Virginia. Evoking the charm of Virginia's colonial estates, this suburban Washington DC hotel combines sophisticated conference facilities, elegant accommodations, and resort activities. The hotel is conveniently located just seven miles from Washington Dulles International Airport and only 30 minutes from downtown Washington DC.

For your computer use while at the Westfields Marriott, wireless internet access is available in public areas and meeting rooms. Wired high speed internet access and local and long distance call service are available for a fee of $9.95 per day in the guest rooms.

Registration

To register online, visit www.mayo.edu/cme/feb2006.html, or complete the attached registration form and return by mail or fax. The registration fee includes tuition, comprehensive course syllabus, continental breakfasts, break refreshments, Thursday evening reception and Friday lunch. The Thursday evening reception is an additional $25 for each guest. Although it is not Mayo School of Continuing Medical Education policy to limit the number of registrants for a course conference, early registration is advised as room facilities may necessitate closing of enrollment. A letter of confirmation will be sent upon receipt of payment and completed registration form. Please present the confirmation letter when checking in at the meeting registration desk.

General Information

Cancellation Policy

If you cancel your participation in this course, your registration fee, less a $75 administrative fee, will be refunded when written notification is received by Mayo School of Continuing Medical Education before February 8, 2006, (FAX: 507 284-0532). No refunds will be made after February 8, 2006.

Mayo School of Continuing Medical Education reserves the right to cancel or postpone any course due to unforeseen circumstances. In the unlikely event that Mayo School of Continuing Medical Education must cancel or postpone this course, Mayo School of Continuing Medical Education will refund the registration fee, but is not responsible for any related costs, charges, or expenses to participants, including fees assessed by airline/travel/lodging agencies.

Lodging Accommodations

Guest rooms have been reserved for attendees and their guests with a special course rate of $159 single/double per night at the Westfields Marriott in Chantilly, Virginia. Hotel room rates are subject to applicable state and local taxes (currently 9%) in effect at the time of check-in. Call Marriott reservations directly at (800) 228-9290 or (703) 818-0300 to reserve your room. To receive the special rate, reservations must be made before the room block is filled or before the expiration date of Thursday, February 2, 2006, whichever comes first. Reservations will be taken following this date based on space and rate availability. Please identify yourself as a participant of the Mayo/FDA sponsored course when making your reservation.

Lodging arrangements are the sole responsibility of the individual registrant.

For additional information about the Westfields Marriott you may view the hotel facts sheet at *http://marriott.com/property/factsheet/iadwf*

Travel

For assistance in making your travel arrangements you may contact Corporate Travel, 800-526-4540/507-282-9121, fax 507-282-9020, or email lori@ctsrst.com. Please reference the Mayo Clinic Continuing Medical Education course "Mayo/FDA CME course" when making travel arrangements. Reduced airfares are subject to availability. To take maximum advantage of discounted rates, it is strongly recommended that flights be booked 60 or more days prior to actual travel.

The estimated taxi fare from Washington Dulles International Airport (IAD) is $20 one way. Ample complementary self-parking is available at the hotel or valet parking is available for a fee of $12 per stay.

Travel arrangements are the sole responsibility of the individual registrant.

Social Programs

Course Reception and Social Event
Thursday, February 23, 2006
6:00 – 9:00 pm

Attendees and their guest(s) are cordially invited to the reception hosted by George and Martha Washington on Thursday, February 23. The event is free for registered course participants. This reception offers you the perfect opportunity to make connections with existing and new colleagues. Pre-registration is requested with a $25 fee for each guest.

~~Hockey~~ Movie Night in Canada
Friday, February 24, 2006
7:00 - 9:30 pm
Lincoln Forum at the Westfields Marriott
(Complimentary)

Attendees and their guests are invited to have a unique evening at the movies with interactive entertainment. Come enjoy the movie and meet the "famous people" who will be attending. Snacks are provided.

Faculty

MAYO CLINIC COLLEGE OF MEDICINE COURSE DIRECTORS:

Rochester, Minnesota

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FDA/CDER
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Cancer Control
American Cancer Society
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Research Triangle Park, NC

Faculty Disclosure

As a provider accredited by ACCME, Mayo Clinic College of Medicine (Mayo School of Continuing Medical Education) must ensure balance, independence, objectivity and scientific rigor in its educational activities. Course director(s), planning committee members, faculty, and all others who are in a position to control the content of this educational activity are required to disclose all relevant financial relationships with any commercial interest related to the subject matter of the educational activity. Safeguards against commercial bias have been put in place. Faculty also will disclose any off-label and /or investigational use of pharmaceuticals or instruments discussed in their presentation. Disclosure of these relevant financial relationships will be published in course materials so those participants in the activity may formulate their own judgments regarding the presentation.

Program Schedule

Thursday, February 23, 2006

11:00 am – 1:00 pm Registration

11:00 am – 1:00 pm Speaker Meeting/Working Lunch (Closed Session)

1:00 pm – 1:30 pm Opening Remarks

1:30 pm – 2:30 pm FDA Summary/Issues

2:30 pm – 4:00 pm

SESSION 1: CONCEPTUAL ISSUES

This session will explore questions such as:

1) What is a PRO concept?
2) What is an adequate conceptual framework?
3) What are the consequences of proceeding with instrument development without a well-established conceptual framework?
4) Do specific conceptual issues vary or evolve by phase of medical product development (i.e., phase I, II, III, or IV clinical trials)?

2:30 pm – 3:00 pm SESSION 1: Draft Manuscript Presentation

3:00 pm – 4:00 pm SESSION 1: Discussion, Audience Input and FDA Feeback

4:00 pm – 4:30 pm Break

4:30 pm – 6:00 pm

SESSION 2: PRO INSTRUMENT SELECTION

This session will explore questions such as:

1) What is the recommended process for instrument selection?
2) When is it appropriate to develop, modify, or adapt a PRO instrument?
3) When is it appropriate to draw individual subscales or items out of assessments to be used separately as study endpoints?
4) If you modify a PRO do you have to redo all validation? What can be used from the original validation?
5) If you select an existing instrument, what do you do if the original development and validation documentation is inadequate?

4:30 pm – 5:00 pm SESSION 2: Draft Manuscript Presentation

5:00 pm – 6:00 pm SESSION 2: Discussion, Audience Input and FDA Feeback

6:00 pm – 9:00 pm **Reception** (additional fee for this event for guests)

Friday, February 24, 2006

8:00 am – 8:30 am Registration, Continental Breakfast

8:30 am – 9:00 am Housekeeping/Announcements

9:00 am – 10:30 am

SESSION 3: PRO INSTRUMENT DEVELOPMENT ISSUES

This session will explore questions such as:

1) What are the basic qualitative research requirements during instrument development? For example, how much preliminary data is required to justify the aggregation of individual items into a domain score? How do you know when you have captured adequately all the requisite sub-concepts?
2) When can a single question serve as a PRO endpoint? When are multiple items required?
3) What is the best method for constructing a summary score?
4) How do you deal with respondent burden?

Program Schedule

Friday, February 24, 2006 - continued

9:00 am – 9:30 am	**SESSION 3: Draft Manuscript Presentation**
9:30 am – 10:30 am	**SESSION 3: Discussion, Audience Input and FDA Feeback**
10:30 am – 11:00 am	**Break**
	Session 4: Validation of PROs ***This session will explore questions such as:*** 1) What evidence of validity must you provide for a PRO assessment you plan to use in a clinical trial? 2) What kinds of validation can be performed concurrently with different types of trials (i.e., phase I, II, III, or IV clinical trials)? 3) How do you validate the equivalence of translations or electronic versions of established questionnaires? 4) What is the most appropriate use of IRT during instrument development and validation?
11:00 am – 11:30 am	**SESSION 4: Draft Manuscript Presentation**
11:30 am – 12:30 pm	**SESSION 4: Discussion, Audience Input and FDA Feeback**
12:30 pm – 1:30 pm	**Lunch Buffet**
1:30 pm – 3:00 pm	**Session 5: Analysis, Interpretation, and Reporting Results Based on PROs** ***This session will explore questions such as:*** 1) What are the analysis landmines with PRO data in clinical trials and how can they be avoided (e.g., missing data, multiplicity, etc)? 2) What are the best (alternative) methods to assess clinical significance of PROs? 3) How should null results be interpreted? 4) How do you present PRO data most effectively in an FDA application? In labeling?
1:30 pm – 2:00 pm	**SESSION 5: Draft Manuscript Presentation**
2:00 pm – 3:00 pm	**SESSION 5: Discussion, Audience Input and FDA Feeback**
3:00 pm – 3:30 pm	**Break**
3:30 pm – 5:30 pm	**Synopsis of Findings (audience feedback, Q&A, discussion)**
7:00 pm – 9:30 pm	**~~Hockey~~ Movie Night in Canada**

Saturday, February 25, 2006

7:30 am – 8:10 am	**Continental Breakfast**
8:10 am – 8:30 am	**Announcements**
8:30 am – 9:30 am	**FDA Response and Comments**
9:30 am – 10:30 am	**Summary Discussion** 1) Outstanding Issues 2) Resolved Issues 3) Methods for Operationalization
10:30 am – 11:00 am	**Break**
11:00 am – 2:00 pm	**Writing Teams Working Lunch (Closed Session)**

REGISTRATION FORM **2006R437**

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Mail or FAX form with payment to:

Mayo School of Continuing Medical Education
Plummer 2-60
200 First Street SW
Rochester, MN 55905

Phone 800-323-2688 or 507-284-2509
FAX 507-284-0532
E-mail cme@mayo.edu
Web site www.mayo.edu/cme

Submit on-line registration at http://www.mayo.edu/cme/feb2006.html

Contact Information

Name of Registrant – *first name, middle name or initial, and last name*		Degree – *select all that apply* ☐ MD ☐ PhD ☐ Other – *specify:*	
Name of Institution		Medical Specialty	
Work/Business Address – *street address*		Work Phone – *include all country and city/area codes as needed along with complete phone number*	
City	State or Province	ZIP or Postal Code	Country
Home Address – *street address*		Home Phone – *include all country and city/area codes as needed along with complete phone number*	
City	State or Province	ZIP or Postal Code	Country
E-mail Address	FAX – *include all country and city/area codes as needed along with complete phone number*	FAX Location – *select one* ☐ Work/Business ☐ Home	

SPECIAL NEEDS If you have special assistance needs or dietary restrictions, describe here:

Events

Reception – *Thursday evening, February 23, 6:00 p.m. – 9:00 p.m.*
☐ I will be attending complimentary
☐ Number of guest(s) _____ @ $25 $ _____

~~Hockey~~ Movie Night in Canada – *Friday evening, February 24, 7:00 p.m. – 9:30 p.m.*
☐ I will be attending complimentary
☐ Number of guest(s) _____ complimentary

Registration Fees

Standard

Postmarked on or before January 13, 2006	$375	$ _____
Postmarked after January 13, 2006 and on site	$425	$ _____

Academic and Government

Postmarked on or before January 13, 2006	$300	$ _____
Postmarked after January 13, 2006 and on site	$350	$ _____

Payment Information

☐ Check is enclosed in the amount shown at right – *make checks payable to Mayo Foundation*			Payment Total
Credit Card – *select one* ☐ Discover ☐ MasterCard ☐ Visa	Account Number	Exp Date – *mm/yy*	
Name of Cardholder – *as it appears on the card*	Signature of Cardholder – *required* **X**		

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If you already received a copy of this brochure, please give this brochure to an interested colleague.