

# Clinical Trial Endpoints: Methods and Practice in Developing Measurements A One-Day Interactive Workshop with Special Emphasis on Rare Diseases

October 25, 2012



## PROGRAM COMMITTEE

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Director for Study Endpoints and Labeling  
Office of New Drugs, CDER  
FDA

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Peninsula College of Medicine and Dentistry, UK

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Consumer Safety Officer, Study Endpoints and Label  
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Endpoints Reviewer, Study Endpoints and Labeling  
Development Staff, CDER- New Drugs  
FDA

### Ashley Slagle

Fellow  
Oak Ridge Institute for Science and Education (ORISE)

## WHO SHOULD ATTEND

This workshop is for Industry, Academia, and Government professionals, both Clinicians and Non-Clinicians, engaged in the development of therapeutic products for all diseases and conditions, especially rare diseases and others for which patient-focused outcomes define appropriate study endpoints.

### Specific roles may include:

- Medical product development teams, including Medical Directors and Chief Medical Officers
- Study endpoints and outcomes researchers
- Instrument developers
- Global health outcomes teams
- Biopharmaceutical and device clinical research professionals
- Clinical operations teams
- Investigative sites,
- All clinical trial personnel
- Regulatory strategy professionals
- Statisticians
- Data analysts

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800 Enterprise Road, Suite 200  
Horsham, PA 19044, USA

### DIA Regional Offices

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## ONE-DAY INTERACTIVE WORKSHOP WITH SPECIAL EMPHASIS ON RARE DISEASES

Clinical Outcome Assessments that measure patient symptoms and functioning are used in clinical trials to evaluate treatment benefit. Good measurement principles are of growing interest in all therapeutic areas because of the need for efficiency in medical product development. This is particularly true for clinical trials in rare diseases that occur in all age groups and are often chronic and disabling. Clinically meaningful outcome measures can be based on patient, caretaker, or clinician input. The usefulness of a measure depends on the targeted context of use. This hands-on workshop, in which participants will have the opportunity to take an active role, will provide a detailed examination of the process for developing, validating, and implementing patient-focused clinical trial outcome measures that will meet regulatory requirements for product approval and labeling.

## SESSION TOPICS

- Attention to Measurement in Clinical Trials: Why it Matters
- Preparing the Groundwork for Clinically Meaningful Measurement: Getting the Content Right
- Generating an Instrument with an Interpretable Score
- Incorporating a Well-Defined and Reliable Measure into an Adequate and Well-Controlled Study

## LEARNING OBJECTIVES

At the conclusion of this workshop, participants should be able to:

- Explain why the application of good measurement principles is necessary to the design of clinical trial outcome measures that meet regulatory requirements for product approval and labeling
- Discuss the concept of “context of use” and why it is important to identifying meaningful measurements and appropriate measurement procedures
- Describe the key steps in developing the conceptual basis for a patient-focused clinical outcome measure that captures treatment benefit
- Describe the key steps in building a clinically meaningful measurement instrument from strong conceptual underpinnings
- Discuss the characteristics of a clinically meaningful measurement instrument
- Apply good measurement principles to the development of clinical trial endpoints.

## CONTINUING EDUCATION CREDITS



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 Postgraduate Institute for Medicine (PIM) and the Drug Information Association. PIM is accredited by the ACCME to provide continuing medical education for physicians.

PIM designates this live activity for a maximum of *7.5 AMA PRA Category 1 Credit(s)*<sup>™</sup>. Physicians should claim only the credit commensurate with the extent of their participation in the activity.



The Drug Information Association is accredited by the Accreditation Council for Pharmacy Education as a provider of continuing pharmacy education. This program is designated for 7.5 contact hours or .75 continuing education units (CEU's). 0286-0000-12-096-L04-P; Type of Activity: Application.



Drug Information Association has been accredited as an Authorized Provider by the International Association for Continuing Education and Training (IACET), 1760 Old Meadow Road, Suite 500, McLean, VA 22102.

As an IACET Authorized Provider, Drug Information Association offers CEUs for its programs that qualify under the ANSI/IACET Standard. Drug Information Association is authorized by IACET to offer .8 CEUs for this program. Participants must attend the entire program in order to be able to receive an IACET statement of credit. No partial credit will be awarded.

If you would like to receive a statement of credit, you must attend the program, sign-in at the DIA registration desk, and complete the on-line credit request process through My Transcript. To access My Transcript, please go to [www.diahome.org](http://www.diahome.org), select "Login to My DIA" and you will be prompted for your user ID and password. Select "My Transcript" (left side bar) and "Credit Request" to process your credit request. Participants will be able to download a statement of credit upon successful submission of the credit request. My Transcript will be available for credit requests on November 8, 2012.

### ACPE Credit Request Update

DIA is required by the Accreditation Council for Pharmacy Education (ACPE) to report pharmacy-requested CEUs through the CPE Monitor system. All ACPE-certified activity credit requests need to be submitted through DIA's My Transcript within 45-days post activity. Pharmacists will need to provide their National Association of Boards of Pharmacy (NABP) e-Profile ID and date of birth (MMDD) to ensure the data is submitted to the ACPE and NABP properly. If you need to obtain your NABP e-Profile, please visit [www.cpemonitor.net](http://www.cpemonitor.net).

### DIA's Certificate Program Statement

This program is part of DIA's Certificate Program and is awarded the following:

- Clinical Research Certificate Program: 4 Elective Units

For more information go to [www.diahome.org/certificateprograms](http://www.diahome.org/certificateprograms)

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The Postgraduate Institute for Medicine (PIM) and DIA require instructors, planners, managers and other individuals who are in a position to control the content of this activity to disclose any real or apparent conflict of interest they may have as related to the content of this activity. All identified conflicts of interest are thoroughly vetted by PIM and DIA for fair balance, scientific objectivity of studies mentioned in the materials or used as the basis for content, and appropriateness of patient care recommendations.

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7:30-8:30 AM REGISTRATION AND CONTINENTAL BREAKFAST

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8:30-8:45 AM WELCOME AND OPENING REMARKS

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8:45-9:15 AM SESSION 1

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### Attention to Measurement in Clinical Trials: Why it Matters

SESSION CHAIR:

#### Laurie Burke, MPH, RPh

Director for Study Endpoints and Labeling  
Office of New Drugs, CDER, FDA

Good measurement principles form the foundation of FDA's regulatory requirements for clinical outcome measures. This introductory session explains why investment in measurement is recommended and how it will ultimately benefit people with diseases, drug developers, clinical trialists, and regulatory authorities. This session will review the regulatory framework for the comprehensive process of creating and implementing a new outcome measure or modifying an existing instrument. This session emphasizes the importance of identifying what is meaningful to be measured as well as how to measure it.

#### Marc K Walton, MD, PhD

Associate Director for Translational Medicine  
Office of Translational Sciences  
CDER, FDA

9:15 AM-12:15 PM SESSION 2

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### Preparing the Groundwork for Clinically Meaningful Measurement: Getting the Content Right

SESSION CHAIR:

#### James P. Stansbury, PhD, MPH

Endpoints Reviewer, Study Endpoints and Labeling Development  
CDER, FDA

The development of a patient-based, clinical outcome measure that will support a labeling claim requires advance planning in the early phases of product development. Establishing the context of use and planning the protocol for qualitative research that thoroughly explores the conceptual basis for measurement are fundamental. Key steps, including identification of the concept and patient population, recruitment of the right range of patients, interviewing, qualitative analysis, item generation and tracking, and development of the draft instrument, will be covered. The session will look at examples of how the key steps have been applied in specific indications, and participants are encouraged to bring their own examples and problems to act as meaningful discussion points.

#### Elektra Papadopoulos

Medical Officer, Office of New Drugs, CDER  
FDA

#### Brian Taylor

Patient Representative

12:15 - 12:45 PM LUNCHEON

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12:45 - 4:30 PM SESSION 3

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### Generating an Instrument with an Interpretable Score

SESSION CHAIR:

#### Jeremy Hobart, PhD FRCP

Professor, Clinical Neurology and Health Measurement  
Peninsula College of Medicine and Dentistry

Session 3 starts where session 2 ends – with a draft instrument – and outlines the stages required to build from this a clinically meaningful instrument with strong psychometric properties that generate interpretable measurements from scores. The session emphasizes the value to efficient scale development of an iterative, mixed method and hypothesis-testing experimental process that begins with perhaps surprisingly small sample quantitative work.

#### Brian Taylor

Patient Representative

4:30 - 5:00 PM SESSION 4

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### Incorporating a Well-Defined and Reliable Measure into an Adequate and Well-Controlled Study

#### Robert Temple, MD

Associate Director, Center for Drug Evaluation and Research  
FDA

This session will provide an overview of important clinical trial design principles to improve the potential for clinically meaningful instruments to detect a treatment effect and support conclusions of treatment benefit. Regulatory requirements for adequate and well-controlled studies include appropriately defined endpoints with adequate planning for interpretation of results in the clinical trial protocol and statistical analysis plan. Important examples of both success and failure will be used to close out the day and provide a basis for a final interactive discussion.

5:00 PM

WORKSHOP ADJOURNED

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# REGISTRATION FORM

Attendees may register online at [www.diahome.org](http://www.diahome.org).



Please complete form in its entirety.

**EVENT TITLE** \_\_\_\_\_  
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**TUTORIAL FEE(S)** \$ \_\_\_\_\_

**MEMBERSHIP:** US \$175

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