



Measuring Study Endpoints in Multinational Clinical Trials: Outcomes Reported from the Viewpoint of the Clinician, Patient, and Caregiver

October 26-27, 2009 | Sheraton New Orleans Hotel, New Orleans, LA, USA

PROGRAM COMMITTEE

LAURIE BURKE, MPH, CAPT. USPHS

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

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JOHN M. WEILER, MD, MBA

President
Compleware Corporation

KEITH W. WENZEL

Senior Product Director, ePRO
Perceptive Informatics®, Inc.

WHO SHOULD ATTEND

This program is designed for

- Biopharmaceutical and device clinical research physicians and medical product development teams
- Clinical operations and global health outcomes teams
- Instrument developers, investigative sites, and other study endpoints researchers
- All clinical trial personnel

This meeting, sponsored by DIA's new Study Endpoints SIAC, is contiguous with and co-located in the same city as ISOQOL's Annual Meeting. It is a forum for interested individuals to drive the future direction of this new DIA SIAC.

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Study endpoints used in clinical trials include, but are not limited to clinician-reported outcomes (ClinROs), patient-reported outcomes (PROs), and reports by caregivers and other observers of patient signs and behaviors related to health status. There has been significant discussion, but little consensus with respect to a common set of best practices applicable across all types of these report-based measures used as study endpoints in clinical trials. This conference brings together key stakeholders to discuss the conceptual, measurement, and practical issues regarding these endpoints when applied to product development. Specifically, the scientific and regulatory issues will be discussed when these types of study endpoints are intended to be used in support of medical product labeling claims.

The conference will begin with a set of presentations to set the context for and to provide a definition and overview of the range of study endpoints used in multinational trials. The conference will include presentations from representatives of the FDA and EMEA who will provide the multinational regulatory perspectives.

Subsequent sessions will include discussion of common principles for the development and validation of all study endpoint measures. The relationships between entry criteria and outcome measurement and between different types of endpoint measures will also be explored.

You are invited to help guide the direction of the DIA's new Study Endpoint SIAC and to interact with industry peers, regulators, instrument developers, clinical research physicians and technology vendors on this important topic.

For conference information, contact

Marjorie Davis | Phone +1-215-442-6176 | email Marjorie.Davis@diahome.org
JoAnn Boileau | Phone +1-215-442-6175 | email Joann.Boileau@diahome.org

THIS PROGRAM WAS DEVELOPED BY THE STUDY ENDPOINTS SPECIAL INTEREST AREA COMMUNITY



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Disclosure Policy: It is Drug Information Association policy that all faculty participating in continuing education activities must disclose to the program audience (1) any real or apparent conflict(s) of interest related to the content of their presentation and (2) discussions of unlabeled or unapproved uses of drugs or medical devices. Faculty disclosure will be included in the course materials.

Learning Objectives: At the conclusion of this conference, participants should be able to:

- ▶ Distinguish between different types of study endpoints
- ▶ Define the range of measure types used as study endpoints to support labeling claims in the US and EU
- ▶ Characterize when ClinRO, PRO, Caregiver or other report-based study endpoint use is most appropriate
- ▶ Explain how to implement ClinRO, PRO, Caregiver or other report-based study endpoints in multinational trials

Unless otherwise disclosed, DIA acknowledges that the statements made by speakers are their own opinion and not necessarily that of the organization they represent, or that of the Drug Information Association. Speakers and agenda are subject to change without notice.

Recording of any DIA tutorial/workshop information in any type of media, is prohibited without prior written consent from DIA.

MONDAY • OCTOBER 26

11:00 AM-1:00 PM REGISTRATION

1:00-1:15 PM WELCOME AND OPENING REMARKS

John M. Weiler, MD, MBA

President
ComplereWare Corporation
Chair of the Study Endpoints SIAC

1:15-3:00 PM SESSION 1

DEFINING TERMS AND SETTING THE REGULATORY CONTEXT

SESSION CHAIRPERSON

Laurie Beth Burke, RPh, MPH

Study Endpoints and Labeling (SEALD), OND, CDER, FDA

This session will provide a general overview of study endpoint use in international medical product development and labeling. Ranges of study endpoint types and clinical trial objectives will be identified with examples from approved products in both the EU and the US. A proposed set of definitions and organizing terminology will be presented. This session will be designed to outline the scope of the following session and to provide the meeting participants with a taxonomy for the discussions planned.

CLINICIAN, PATIENT, AND CAREGIVER REPORTS: WHAT'S THE SAME AND WHAT'S DIFFERENT?

Ann Marie Trentacosti, MD

Endpoints Reviewer, SEALD
OND, CDER, FDA

CLINICIAN, PATIENT, AND CAREGIVER REPORTS: WHAT CAN WE LEARN FROM APPROVED LABELING IN THE US?

Elektra Papadopoulos, MD

Endpoints Reviewer, SEALD
OND, CDER, FDA

CLINICIAN, PATIENT, AND CAREGIVER REPORTS: WHAT CAN WE LEARN FROM APPROVED LABELING IN THE EU?

Mira Pavlovic, MD

Head of Scientific Advice Unit at AFSSAPS, Paris, France

3:00-3:30 PM REFRESHMENT BREAK, TABLE TOP EXHIBITS, AND POSTERS

3:30-5:30 PM SESSION 2

MODELS, MEASURES AND CLAIMS

SESSION CHAIRPERSON

Donald Patrick, PhD, MSPH

Professor
University of Washington

Relationship of patient, clinician and caregiver perspectives to physiologic and survival endpoints. Brief reports from different disorders and endpoints (e.g. IBS, Insomnia, RA, Depression Sleep). What is the purpose of PRO and ClinRO use in drug and device development? Endpoint examples in cases where there is no physiologic endpoint—have to use PRO or observer, co-primary, secondary endpoint, risk assessment.

THE TROUBLE WITH GLOBALS – ECOG/KARNOFSKY

Amy Abernethy, MD

Director, Duke Cancer Care Research Program
Duke University

CLINICIAN RATING SCALES IN CLINICAL TRIALS

Jeremy Hobart, MD

Senior Lecturer and Honorary Consultant Neurologist
Neurology Research Group, UK

AE REPORTING – A PATIENT OR A CLINICIAN ASSESSMENT?

Ethan Basch, MD, MSc

Health Outcomes Group
Departments of Medicine and Biostatistics
Memorial Sloan-Kettering Cancer Center

5:30-7:30 PM RECEPTION AND EXHIBITS: DISCUSSANT ON POSTER SUBMISSIONS

8:30-10:00 AM SESSION 3

EVALUATING AND DEMONSTRATING CONTENT VALIDITY – PART 1: WHAT HAVE WE LEARNED FROM PROs?

SESSION CHAIRPERSONS

Nancy Kline Leidy, PhDSenior Vice President, Scientific Affairs
United BioSource Corporation**Jean Paty, PhD**Founder & Senior Vice President
Scientific Quality & Regulatory Affairs
invivodata, Inc.

This session will address content validity in the context of the regulatory environment and medical product development, with specific reference to lessons learned from the field of patient-reported outcomes (PRO). The session will begin with an overview of content validity, including how it is defined; its relationship to the determination, selection, and positioning of study endpoints; its role in instrument evaluation and selection; and potential threats to content validity. Content of this first presentation will be drawn from the psychometric literature and the report of the ISPOR Task Force on content validity in the use and modification of existing instruments. The second presentation will describe methodological issues in content validity, with examples drawn from the development and evaluation of PRO instruments. The presentation will include key issues in the conduct of focus groups, 1:1 interviews, and cognitive interviews, indicators of saturation, and international considerations and include key points from the ISPOR Task Force on evaluating and documenting content validity in PROs. The third presentation will address the issue of concept clarification for clinician-reported outcomes, including issues such as clinical definitions, differential diagnosis versus severity assessment, and the standardization of symptom evaluation. The session will conclude with a panel discussion of regulatory issues in content validity with reference to the preceding presentations and questions from the audience.

OVERVIEW OF CONTENT VALIDITY: WHAT IS IT? WHY IS IT IMPORTANT? WHEN IS IT AT RISK?**Nancy Kline Leidy, PhD**Senior Vice President, Scientific Affairs
United BioSource**METHODOLOGICAL ISSUES IN CONTENT VALIDATION****Mona L. Martin, RN, MPA**Executive Director
Health Research Associates**CLINICIAN PERSPECTIVE OF CONTENT VALIDITY: DIAGNOSES, DEFINITIONS AND SYMPTOMS – EXAMPLES FROM GI****Nimish Vakil, MD, FACP, FACP**Clinical Professor of Medicine, University of Wisconsin
Medical School, College of Health Sciences,
Marquette University

PANEL DISCUSSION

REGULATORY ISSUES IN CONTENT VALIDITY

PANELISTS

Elektra Papadopoulos
Ann Marie Trentacosti
Laurie Burke
Mira Pavlovic

10:00-10:30 AM REFRESHMENT BREAK AND EXHIBITS

10:30 AM-12:00 PM SESSION 4

EVALUATING AND DEMONSTRATING CONTENT VALIDITY – PART 2: THE CASE OF CAREGIVER AND CLINICIAN-REPORTED OUTCOMES

SESSION CHAIRPERSONS

Nancy Kline Leidy, PhDSenior Vice President, Scientific Affairs
United BioSource Corporation

This session will build on the information presented in Part 1 by discussing content validity in pediatric, caregiver, and clinician-reported outcomes. The session will begin with a discussion of pediatric-reported outcomes, drawing from the paper by Matza et al (2004, Value in Health 7(1), 79-92) and recent work of the ISPOR task force on pediatric outcomes assessment. The second presentation will address caregiver-reported outcomes using examples drawn from assessments of patients with cognitive impairment. The third presentation will address content validity issues in clinician-reported outcomes. The session will conclude with a panel discussion of these types of endpoints from a regulatory perspective.

CONTENT VALIDITY IN PEDIATRIC ASSESSMENT**Louis Matza, PhD**Research Scientist
Center for Health Outcomes Research
United BioSource Corporation**CONTENT VALIDITY IN CAREGIVER-REPORTED OUTCOMES FOR COGNITIVE IMPAIRMENT****Lori Frank, PhD**Executive Director
Center for Health Outcomes Research
United BioSource Corporation**CONTENT VALIDITY AND CLINICIAN-REPORTED OUTCOMES: PAST, PRESENT, & FUTURE****John Powers III, MD, FACP, FIDSA**Clinical Assistant Professor of Medicine
School of Medicine
George Washington University

PANEL DISCUSSION

REGULATORY PERSPECTIVE OF CONTENT VALIDITY IN PEDIATRIC, CAREGIVER, AND CLINICIAN-REPORTED OUTCOMES

PANELISTS

Ann Marie Trentacosti
Elektra Papadopoulos
Laurie Burke
Mira Pavlovic

12:00-1:00 PM LUNCH IN EXHIBIT HALL

1:00-3:00 PM SESSION 5

PRACTICAL CONSIDERATIONS FOR MULTI-NATIONAL CLINICAL TRIALS

SESSION CHAIRPERSONS

John M. Weiler, MD, MBAPresident
CompleWare Corporation

Keith W. Wenzel

Senior Product Director, ePRO
Perceptive Informatics, Inc.

As study endpoints are applied in international clinical trials, there are important practical considerations that affect their use. This session will discuss implementation of study endpoints and the factors that must be considered in multi-national trials including instrument development and modification, international harmonization, and technology. Six subject matter experts will present in a "rapid fire," 20 minute presentation format. At the end of the session, the audience will have gained practical insight about the implementation of study endpoints in multi-national clinical trials.

INSTRUMENT DEVELOPMENT FOR MULTI-NATIONAL APPLICATION**Ingela Wiklund, PhD**

Senior Research Leader
Center for Health Outcomes Research
United BioSource Corporation

**ENDPOINTS BASED ON PATIENT REPORTED OUTCOMES:
IMPLEMENTATION IN GLOBAL CLINICAL TRIALS****Catherine Milch, MD**

Medical Director
Millennium Pharmaceuticals

**C-PATH'S PRO CONSORTIUM. A PUBLIC-PRIVATE PARTNERSHIP
TO FACILITATE PRO STANDARDIZATION****Stephen Joel Coons, PhD**

Director
PRO Consortium

**FACILITATING STUDY ENDPOINT DATA COLLECTION VIA
TECHNOLOGY****Keith Wenzel**

Senior Product Director, ePRO
Perceptive Informatics, Inc.

**A PHYSICIAN'S VIEW OF THE IMPORTANCE OF METHODS USED
FOR REPORT-BASED MEASURES IN MULTINATIONAL CLINICAL
TRIALS****Valdo Arnera, MD**

General Manager, Europe
PHT Corporation

INDUSTRY BEST PRACTICE FOR INSTRUMENT MODIFICATION**Katrina Halling**

PRO Consulting

3:00-3:30 PM**REFRESHMENT BREAK AND TABLE TOP
EXHIBITS****3:30-5:30 PM****SESSION 6****DEFINING A RESEARCH AGENDA FOR STUDY ENDPOINT
MEASUREMENT AND MULTI-NATIONAL CLINICAL TRIALS**

SESSION CHAIRPERSON

Jay Pearson, PhD

Senior Director, Scientific Staff
Epidemiology Department
Merck Research Laboratories

This session will facilitate a collaborative discussion of a research agenda for Study Endpoint methodology. During the first hour, the audience will divide into three breakout groups (patient-reported outcomes, clinician-reported outcomes, and observer-rated outcomes) to discuss the key methodological or theoretical gaps with respect to content validity, measurement, claims language, and conduct of multinational trials. The second hour will consist of brief presentations of the key discussion points by the facilitators from each breakout group and an interactive discussion of these points among the combined audience and a panel of the meeting session chairs.

3:30-4:30 PM**BREAKOUT SESSION**

FACILITATORS:

Program Committee members

Breakout sessions to discuss key methodological and theoretical gaps with respect to content validity: ClinRO, PRO, Caregiver/other

4:30-5:30 PM**SUMMARY OF BREAKOUT SESSION**

- Discussion panel
- Audience participation
- Identification of Next Steps

5:30 PM

CONFERENCE ADJOURNED

TRAVEL AND HOTEL

The most convenient airport is New Orleans International Airport and attendees should make airline reservations as early as possible to ensure availability. The Sheraton New Orleans Hotel is holding a block of rooms at the reduced rate below until September 28, 2009, for the DIA event attendees. Room availability at this rate is guaranteed only until this date or until the block is filled.

Single \$205+**Double \$205+**

Please contact the Sheraton New Orleans Hotel by telephone at +1-504-525-2500 and mention the DIA event. Callers should refer to the ISOQOL/DIA room block to attain discounted rate. The hotel is located at 500 Canal Street, New Orleans, LA 70130, USA.

GROUP DISCOUNTS*

Register 3 individuals from the same company and receive complimentary registration for a 4th! **All 4 individuals must register and prepay at the same time – no exceptions.** DIA will apply the value of the lowest applicable fee to this complimentary registration; it does NOT include fees for optional events or DIA membership. You may substitute group participants of the same membership status at any time; however, administrative fees may be incurred. **Group registration is not available online and does not apply to the already-discounted fees for government or charitable nonprofit/academia.**

- ▶ To take advantage of this offer, please make a copy of this registration form for EACH of the four registrants from your company. Include the names of all four group registrants on each of the forms and return them together to DIA.

Participants with Disabilities:

DIA event facilities and overnight accommodations are accessible to persons with disabilities. Services will be made available to sensory-impaired persons attending the event if requested at least 15 days prior to event. Contact the DIA office to indicate your needs.

Measuring Study Endpoints in Multinational Clinical Trials:

Outcomes Reported from the Viewpoint of the Clinician, Patient, and Caregiver

Event ID #09025

Sheraton New Orleans Hotel

New Orleans, LA, USA

OCTOBER 26-27, 2009

Sponsored by DIA's
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Register online or fax this page to +1-215-442-6199

▶ CONTACT & TABLETOP EXHIBIT INFORMATION

Attendees may visit the tabletop exhibits during the event and during receptions (if applicable).

Event information: Contact JoAnn Boileau at the DIA office by telephone +1-215-442-6175, fax +1-215-442-6199 or email JoAnn.Boileau@diahome.org.

Tabletop exhibit information: Contact Shannon Lewis, Exhibits Associate, at the DIA office by telephone +1-215-442-6149, fax +1-215-442-6199 or email Shannon.Lewis@diahome.org. For tabletop exhibit space, please check the box below.

To receive a tabletop exhibit application, please check.

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Registration Fees If DIA cannot verify your membership upon receipt of registration form, you will be charged the nonmember fee. Registration fee includes refreshment breaks, luncheons, and reception (if applicable), and will be accepted by mail, fax, or online.

Member Fee US \$1100

Join DIA now to qualify for the early-bird member fee! www.diahome.org/en/Membership/AboutMembership/AboutMembership

MEMBERSHIP
US \$ 140

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Nonmember Fee US \$1240

A one-year membership to DIA is available to those paying a NONMEMBER registration fee. If paying a nonmember fee, please indicate if you do, or do not, want membership.

I want to be a DIA member I do NOT want to be a DIA member

Discount Fees	MEMBER	NONMEMBER*
Government (Full-time)	US \$ 365 <input type="checkbox"/>	US \$ 505 <input type="checkbox"/>
Charitable Nonprofit/Academia (Full-time)	US \$ 680 <input type="checkbox"/>	US \$ 820 <input type="checkbox"/>

*If paying a nonmember fee, please check one box above, indicating whether you want membership.

To receive a tabletop exhibit application, please check.

▶ CANCELLATION POLICY: On or before OCTOBER 19, 2009

Administrative fee that will be withheld from refund amount:

Member or Nonmember = \$200
Government or Academia or Nonprofit (Member or Nonmember) = \$100
Tutorial (if applicable) = \$50

Cancellations must be in writing and be received by the cancellation date above. Registrants who do not cancel by that date and do not attend will be responsible for the full registration fee paid. Registrants are responsible for cancelling their own hotel and airline reservations. You may transfer your registration to a colleague at any time but membership is not transferable. Please notify DIA of any such substitutions as soon as possible. Substitute registrants will be responsible for nonmember fee, if applicable.

▶ DIA reserves the right to alter the venue, if necessary. If an event is cancelled, DIA is not responsible for any airfare, hotel or other costs incurred by registrants.

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BANK TRANSFER When DIA completes your registration, an email will be sent to the address on the registration form with instructions on how to complete the Bank Transfer. Payment should be made in US dollars. Your name and company, as well as the Event I.D. # must be included on the transfer document to ensure payment to your account.