Beginning with the End in Mind – Study Endpoints: Targeting Patient-Centered Outcomes

October 21–23 | North Bethesda, MD

PROGRAM CO-CHAIRS:

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

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OVERVIEW:

Study endpoint measures in clinical trials determine what conclusions can be made about treatment benefit, medical differentiation, and value. For medical product developers, this evidence plays a critical role in drug development futility decisions as well. Measure selection varies depending upon the development phase and the specific objectives of the trial. Balancing the measurement objectives with various drug development stakeholder interests requires thoughtful planning and consideration. Evidence requirements to support labeling and promotion claims can require substantial time and effort to coordinate, especially when endpoint measures must be developed de novo. During this meeting, you will gain insight into the tradeoffs and various stakeholder perspectives for developing a study endpoint measurement strategy, including detailed and practical tips for ensuring that measurement tools are adequate to support the targeted objectives with a focus on establishing instrument content validity for the specified clinical trial context of use.

CUSTOMIZE YOUR MEETING EXPERIENCE!

You can customize how many days you attend this meeting, one, two, or three. Build your meeting to suit your needs!

Agenda:

- Day One/Day Two AM: Review the scientific methods, issues, and execution challenges for internal and external stakeholders
- Day Two PM: Discuss measurements to achieve labeling and promotion claims
- Day Three: FDA will present good measurement principles ensuring that the measure is meaningful and interpretable

LEARNING OBJECTIVES:

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

- Recognize the challenges Sponsors face with setting an endpoint strategy and prioritizing the endpoint objectives across multiple internal stakeholders.
- Discuss the scientific methods, issues and execution challenges for each of the respective internal and external stakeholder's endpoint goals.
- Populate and employ strategic drug development tools such as the Target Product Profile to facilitate study endpoint objectives and regulatory interactions.
- Discuss the characteristics of a clinically meaningful measurement instrument.
- Apply good measurement principles to the development of clinical trial endpoints.
- Discuss why the "context of use" is important to developing, evaluating, and interpreting meaningful measurements and appropriate measurement procedures.
- Describe the key steps in meaningful measurement starting with establishing the conceptual basis for a patient-focused clinical outcome measure of treatment benefit.

Register at **diahome.org/studyendpoints**

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October 22 (pm only) and October 23 (1.5 day attendance): 1.0 IACET CEUs
October 21-22 (2-day attendance): 1.3 IACET CEUs
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Day 1 (October 21): 6.5 contact hours or .65 CEUs, 0286-0000-13-088-L04-P
Day 2: am Sessions (October 22): 3.5 contact hours or .35 CEUs, 0286-0000-13-089-L04-P
Day 2: pm Sessions (October 22): 3.25 contact hours or .325 CEUs, 0286-0000-13-090-L04-P
Day 3 (October 23): 6.75 contact hours or .675 CEUs, 0286-0000-13-091-L04-P

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MONDAY, OCTOBER 21

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

How Study Endpoints are Used and Why

Linda S. Deal, MS
Clinical Outcomes Assessment (COA) Director
Health Economics Outcomes Research and Epidemiology
Shire

8:45-9:30 AM

Commercials Interest in Study Endpoints

Joseph Caminiti
President and Chief of Staff
BQ6 Media Group

- The value of pharmaceutical agents and treatments depends on the proven efficacy, safety, and quality of care versus an accepted comparator.
- There are potential differences in demonstrating efficacy and safety vs. suitability for P&T inclusion and/or reimbursement.
- Commercialization involves leveraging the intrinsic properties of a compound to develop promotional strategies around the unique product attributes that provide value for physicians, patients, and payers
- Exploiting these "unique" properties with product promotion and sales support activities that support successful market launch and growth needs to be supported by data.

9:30-10:15 AM

Translational Medicine: The Bridge Between the Beginning and the End

Francisco Leon, MD, PhD
Vice President, Translational Medicine, Immunology Development
Janssen R&D (Pharmaceutical Companies of Johnson & Johnson)

Translational Medicine bridges pre-clinical and clinical development. "Reverse Translation" planning often results in recommendations for the development of patient-reported outcomes and biomarkers in Early Development. Challenges and case studies will be discussed.

10:15-10:30 AM REFRESHMENT BREAK

10:30-11:15 AM

Addressing Study Endpoints for Clinical Relevance

Debra Silberg, MD, PhD
Senior Director, Clinical Development
Shire

Diseases can be evaluated by changes in physiology, signs and symptoms. When performing a clinical study, it is important to determine the endpoint that is most clinically relevant, discussing with both clinicians and patients what they are looking for from the treatment. The view may not always be the same, but should be taken into consideration when evaluating efficacy. This talk will discuss different endpoints from the viewpoint of both the patient and clinician.

11:15 AM-12:30 PM

Study Endpoints: A Market Access Perspective

Mohan Bala, PhD
Head Oncology Value & Access
Sanofi

- Overview of Market Access process and evolving concept of Value
- Relevance of study endpoints for Market Access
- Payer perspective on endpoints from key countries: A few illustrative examples
- Study endpoints and Market Access: what does the future hold?

12:30-1:30 PM LUNCH

1:30-2:00 PM PANEL DISCUSSION

What Does Differentiation Mean to You and How You Select Endpoints?

MODERATOR

Harris Kaplan
Managing Partner
Red Team Associates, Div Of Healogix

- All Stakeholders want differentiation – are we talking about the same thing?
- What is(are) the priority(ies)? How are these determined/traded off? Are there specific scenarios that influence this e.g. first in class, best in class, second generation of same mechanism of action (i.e. potential generic competition), etc.?
- Speaker representatives of the Stakeholder functions to participate
- The regulatory tight rope

2:00-3:15 PM

Tools to Help Gain Alignment in the Project Team

Charles Gombar, PhD
Senior Vice President, Project Management and Pharmaceutical Development
Endo Pharmaceuticals

There is more than one way to develop most new drugs. A key challenge in pharmaceutical R&D is establishing and maintaining alignment on the development strategy and plan in the project team. This talk will highlight some tools that can be used to ensure that a development plan is aligned with the value proposition for the new product.

3:15- 3:30 PM REFRESHMENT BREAK

3:30-4:15 PM

Let's Talk About Timing and Study Endpoints

Evan Loh, MD
Chairman, Board of Directors
Chief Medical Officer
Paratek Pharmaceuticals

4:15-4:45 PM WRAP UP AND SUMMARIZE THE DAY

Preview of Day 2

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TUESDAY, OCTOBER 22

8:30-8:45 AM WELCOME REFRESH OF DAY 1 AND INTRODUCTION OF DAY 2 AGENDA

What Does it Take to Achieve the Study Endpoint Goals Discussed in Day 1?

Linda S. Deal, MS
Clinical Outcomes Assessment (COA) Director
Health Economics Outcomes Research and Epidemiology
Shire

8:45-9:30 AM

Adaptive Designs and Endpoint Selection

Scott Berry, PhD
President and Senior Statistical Scientist
Berry Consultants

The selection of endpoints in the learn phases of trials can be a very difficult problem typically involving the time of the trial, the relevance of the endpoint, and the power of the study. Many times decisions to select short term endpoints, for the sake of speed of getting to phase III can create poor dose selection, incorrect go/no-go decisions, and inefficient drug development. The issue can be more complex in an adaptive trial design because of the need to provide information for the adaptations. A strength of the adaptive design is that it can utilize endpoints in different ways — early markers can be used as possibly correlated to the more appropriate long-term endpoint, and this correlation can be modeled and informed by the trial, and thus the design can be made more efficient and yet the focus is on the more relevant long-term clinical endpoint.

In this talk the selection of endpoints, the modeling of different endpoints, within the building and simulation of an adaptive clinical trial will be discussed. Real examples of endpoint selection within adaptive designs will be presented.

9:30-10:15 AM

Overcoming Regulatory Challenges in Targeting Patient-Centered Outcomes in Psychiatric Drug Development

Thomas Laughren, MD
Director
Laughren Psychopharm Consulting, LLC.

Selecting the primary measure for a definitive trial and defining the primary study endpoint are critical steps in any drug development program. There is increasing interest in using patient reported outcome (PRO) measures in registration trials, and these measures present many challenges, including regulatory challenges. Included among the regulatory challenges are redundancy with investigator rated measures and pseudo-specificity. This talk will discuss possible approaches to overcoming these and other regulatory challenges associated with PROs.

10:15–10:45 AM REFRESHMENT BREAK

10:45-11:30 AM

Patient-Centered Outcomes Targeting Payers and Regulators

Ethan M. Basch, MD
Director
Cancer Outcomes Research Program
Associate Professor of Medicine
University of North Carolina at Chapel Hill

Payers seek information about the comparative value of products, and increasingly consider the patient experience as an important component of value. Patient-reported outcome (PRO) data may be desired or requested by payers including information about symptoms, functional status, harms of care, treatment preferences, and/or quality of life. There is some overlap with the informational needs and published guidance of regulators. New standards for PROs have also emerged from several entities involved with evaluating comparative effectiveness and quality of care.

11:30 AM-12:15 PM

Study Endpoints and Patient-Centered Outcomes: Payer Needs vs Regulatory Limits

Paul Radensky, MD
Partner
McDermott Will & Emery LLP

Payers are increasingly asking for value information to support coverage and pricing decisions about new drugs and medical devices. Sometimes the data payers want do not fit squarely within the labeling of the medical product. Many were hopeful that Section 114 of FDAMA and the guidance on Patient-Reported Outcomes would reduce uncertainty about what manufacturers could communicate to payers about their products, but much uncertainty—and potential risk—remains. This talk will explore the regulatory landscape for payer communications and consider some options to bridge payer needs and regulatory limits.

12:15-12:30 PM Q & A

12:30-1:30 PM LUNCH

Measurement to Achieve Labeling and Promotion Claims

Good measurement principles form the foundation for clinical outcome measurement. Investment in measurement will ultimately benefit people with diseases, drug developers, clinical trialists, and regulatory authorities. This session will provide an overview of the comprehensive process of creating and implementing a new outcome measure or modifying an existing instrument. The development of a patient-based, clinical outcome measure that will support a labelling 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. This session emphasizes the importance of identifying what is meaningful to be measured based on the context of use for measurement, getting the content right, and finally, ensuring that the measure is meaningful and interpretable.

1:30-3:00 PM

Navigating Instrument Development Using the Wheel and Spokes

Session Chair

Jessica Voqui, PharmD, MS
Endpoints Reviewer, Study Endpoints and Labeling Development
CDER, FDA

This session will give an overview of the process of instrument development as illustrated by the Wheel and Spokes diagram. Responses by EMA and OPDP representatives will explain the relevance of this diagram to their regulatory settings.

Road Map to Patient Focused Outcome Measurements in Clinical Trials

Capt. Laurie Burke, MPH, RPh
Associate Director for Study Endpoints and Labeling
Office of New Drugs
CDER, FDA

EMA Response (Via Telecommunications)

Maria Isaac, MASc, MD, PhD
Scientific Administrator
European Medicines Agency, European Union (EMA)

OPDP Response

Elaine Hu Cunningham, PharmD
Senior Regulatory Review Officer, OPDP
CDER, FDA

Panel Discussion

Moderator

Jessica Voqui

Panelists

Laurie Burke

Maria Isaac

Elaine Hu Cunningham

3:00–3:15 PM REFRESHMENT BREAK

3:15–4:45 PM

Spoke 1: Defining the Clinical Trial Context of Use

SESSION CHAIR

Ashley F. Slagle, MS, PhD
Endpoints Reviewer, Study Endpoints and Labeling Development
CDER, FDA

Good measurement depends on a clearly defined context of use, including explicit consideration of the targeted disease definition, patient population, clinical trial design and objectives, clinical practice, and other aspects of the study setting.

Context of Use: Starting with the Patients — FDA's Patient Focused Drug Development Program

Theresa Mullin, PhD
Associate Director, Office of Planning and Informatics,
CDER, FDA

Context of Use: Industry Perspective

Debra Silberg, MD, PhD
Senior Director, Clinical Development
Shire

Context of Use: Patient Advocate Perspective

Cynthia Bens, MBA
VP, Public Policy, Accelerate Cure/Treatments for
Alzheimer's Disease (ACT-AD)
Alliance for Aging Research

Panel Discussion

MODERATOR

Ashley Slagle

PANELISTS

Theresa Mullin

Debra Silberg

Cynthia Bens

4:45-5:00 PM WRAP-UP AND ADJOURNMENT OF THE DAY

5:00-6:00 PM NETWORKING RECEPTION

A Model of Patient, Payer, and Product Developer Collaboration to Support Innovating for Value

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WEDNESDAY, OCTOBER 23

8:30-8:45 AM WELCOME REFRESH OF DAY 2 AND INTRODUCTION OF DAY 3 AGENDA

Attention to Measurement in Clinical Trials: Why it Matters

Capt. Laurie Burke, MPH, RPh
Associate Director for Study Endpoints and Labeling
Office of New Drugs
CDER, FDA

8:45 AM-10:15 AM

Conceptualization and Generation of a Draft Measure

SESSION CHAIR

Elektra Papadopoulos, MD, MPH
Medical Officer, Study Endpoints and Labeling Development
CDER, FDA

Along with a clearly defined context of use, clear conceptualization provides the foundation for the process of instrument development. This is particularly important in clinical trials for regulatory purposes because the concept of interest becomes the labeling claim. Once there is clarity in conceptualization, the qualitative research process may begin.

Spoke 1: Conceptualization and Making the Case for Content Validity

Donald Patrick, PhD, MPH
Professor, Health Services, School of Public Health
University of Washington

Spoke 2: Interview, Qualitative Analysis, and Item Development Techniques

Ashley Slagle, PhD, MS
Endpoints Reviewer, Study Endpoints and Labeling Development
CDER, FDA

James Stansbury, PhD, MPH
Consumer Safety Officer, Study Endpoints and Labeling Development
CDER, FDA

10:15-10:45 AM REFRESHMENT BREAK

10:45 AM-12:00 PM

Spoke 2: Interview, Qualitative Analysis, and Item Development Techniques continued

Ashley Slagle, PhD, MS
Endpoints Reviewer, Study Endpoints and Labeling Development
CDER, FDA

James Stansbury, PhD, MPH
Consumer Safety Officer, Study Endpoints and Labeling Development
CDER, FDA

Panel Discussion

MODERATOR

Elektra Papadopoulos

PANELISTS

Ashley Slagle

James Stansbury

Cynthia Bens

Donald Patrick

12:00-1:00 PM LUNCHEON

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1:00-3:00 PM

Confirming Content Validity: Finalizing a Measure with an Interpretable Score

SESSION CHAIR

Sarrit Kovacs, PhD
Endpoints Reviewer, Study Endpoints and Labeling Development
CDER, FDA

The afternoon session builds from the basis of the well-conceptualized draft instrument, looking at methods for finalizing a clinically meaningful instrument that provides interpretable measurement in a specific context of use. The session focuses on exploratory use of mixed methods that incorporate quantification to refine meaning and content.

Spoke 2 continued:

Jeremy Hobart, PhD, FRCP
Professor, Clinical Neurology and Health Measurement
Peninsula College of Medicine and Dentistry
United Kingdom

Panel Discussion

MODERATOR

Sarrit Kovacs

PANELISTS

Jeremy Hobart

Ashley Slagle

James Stansbury

Cynthia Bens

3:00-3:15 PM REFRESHMENT BREAK

3:15-4:45 PM

Spoke 3 and 4: Completing the Dossier: Reliability, Construct Validity, Ability to Detect Change, and Interpretation Metrics

SESSION CHAIR

Paivi Miskala, MSPH, PhD
Endpoints Reviewer, Study Endpoints and Labeling Development
CDER, FDA

After content validity is demonstrated, other measurement properties specific to the context of use, need to be demonstrated. This session is dedicated to research methods for identifying the other measurement properties—reliability, construct validity, and the ability to detect change. In addition the metrics of clinically meaningful change in the particular context of use will be discussed.

Practical Consideration when Planning the Evaluation of Measurement Properties

SPEAKER

Patrick Marquis, MD, MBA
President
TwoLegs Consulting, LLC

Longitudinal Psychometric Evaluation

SPEAKER

Laura Lee Johnson, PhD
Statistician, National Center for Complimentary and Alternative Medicine
National Institutes of Health

Interpretation of Scores

SPEAKER

Lisa Kammerman, PhD
Master Reviewer, Office of Biostatistics
CDER, FDA

Panel Discussion

MODERATOR

Paivi Miskala

PANELISTS

Cynthia Bens

Laurie Burke

Jeremy Hobart

Laura Lee Johnson

Lisa Kammerman

Sarrit Kovacs

Patrick Marquis

4:45-5:00 PM WRAP UP

Capt. Laurie Burke, MPH, RPh
Associate Director for Study Endpoints and Labeling
Office of New Drugs
CDER, FDA

5:00 PM MEETING ADJOURNED