DIA Advancing the Science of Study Endpoints Conference

December 5-6 | Omni Shoreham Hotel | Washington, DC

PROGRAM CHAIRS

Chad Gwaltney, PhD
Principal Consultant
Gwaltney Consulting

Annabel Nixon, PhD
Director
Chilli Consultancy, United Kingdom

Keith W. Wenzel
Senior Director
Perceptive Partner Program

PROGRAM COMMITTEE

René Allard, PhD
Public Disclosure Lead
Grünenthal GmbH, Germany

J. Jason Lundy, PhD
Principal
Outcometrix

David H. Schubert
Vice President of Regulatory and Quality
Stealth BioTherapeutics

Ashley F. Slagle, PhD, MS
Principal, Scientific and
Regulatory Consultant
Aspen Consulting, LLC

Michael Lees
Group Director, WWHEOR
Markets (Oncology)
Bristol-Myers Squibb, United Kingdom

Overview

DIA's conference on clinical trial endpoints will bring together key stakeholders to address critical questions and generate potential solutions to challenges associated with determining study endpoints and outcomes. Examine global strategies for selecting study endpoints, and the impact of study endpoints during analysis of clinical evidence in the various types of drug approval processes.

Who Should Attend

This conference is for industry, academia, government, vendors, clinicians, and health technology agency professionals involved in setting, executing, or evaluating endpoint strategy for drug approval, labeling, promotion, translational science, and market access.

Highlights

Keynote Speakers

Dr. Janet Woodcock
Director of the Center for Drug
Evaluation and Research at FDA

Dr. Mark McClellan
Director of the Duke-Robert J. Margolis, MD,
Center for Health Policy at Duke University

- Global speakers from regulatory agencies, industry, academia, and non-profit organizations
- DIA-ISPOR Session: Prospectively Planning Adaptive Endpoints and Involving All Stakeholders
- A moderated discussion on the use of wearables in clinical trials

This program has been developed in collaboration with the DIA Study Endpoints Community.

Message from Program Co-Chairs

Dear Colleagues,

On behalf of the Program Committee, we are pleased to welcome you to DIA's *Study Endpoints Conference.* We are honored to chair this highly anticipated and invaluable event.

This conference is unique in setting the stage for an open, collaborative discussion of important topics related to both strategic and methodological/scientific considerations for study endpoints among global representatives from industry, academia, nonprofit organizations, and regulatory agencies.

We will kick off the conference with two exciting keynote speakers addressing their persepctives on the practice of study endpoints: *Where are We Now and Where are We Going?* Our first address will be given by the FDA's Director of the Center for Drug Evaluation and Research (CDER), Dr. Janet Woodcock, who will immediately be followed by Dr. Mark McClellan, Director, Duke-Robert J. Margolis, MD, Center for Health Policy at Duke University.

We hope you will take advantage of the many opportunities to actively engage in discussions and with each other. Be sure to join us Monday evening for the Networking Reception.

Best Regards,

Chad Gwaltney, PhD
Principal Consultant
Gwaltney Consulting

Annabel Nixon, PhD
Director
Chilli Consultancy, United Kingdom

Schedule At-A-Glance

DAY ONE | MONDAY, DECEMBER 5

12:00-5:30PM	Registration
1:00-1:25PM	**Welcome and Opening Remarks:** Setting the Stage
1:25-2:40PM	**Session 1: Keynote Addresses:** Perspectives on Study Endpoints: Where are We Now and Where are We Going?
2:40-3:00PM	Refreshment and Networking Break
3:00-4:30PM	**Session 2:** Approval Pathways and Endpoint Selection
4:30-5:30PM	**Session 3:** Research Presentations
5:30-6:30PM	Networking Reception

DAY TWO | TUESDAY, DECEMBER 6

7:30AM-5:00PM	Registration
7:15-8:15AM	Continental Breakfast and Networking
8:15-8:30AM	**Welcome to Day Two**
8:30-9:30AM	**Session 4:** DIA-ISPOR Session: Prospectively Planning Adaptive Study Designs and Involving All Stakeholders
10:00-11:30AM	**Session 5:** Addressing Multiple Stakeholder Needs with COAs and Endpoints Selection
11:30AM-1:00PM	Luncheon
1:00-2:30PM	**Session 6:** Meaningful Score Changes to Establish Endpoint Definitions
3:00-4:30PM	**Session 7:** Clinical Trial Grade Wearables – The Current State of the Science
4:30-5:00PM	**Closing Summary**

Learning Objectives

At The conclusion of this conference, participants should be able to:

- Describe the relationship between endpoint selection and the different types of drug approval pathways
- Discuss the needs and requirements of critical stakeholders — patients, regulatory agencies, clinicians, payers — when identifying endpoints
- Identify techniques for establishing the clinical relevance of changes in endpoints in clinical trials
- Explain the use of wearables for collecting study endpoint data in clinical trials

Continuing Education Credit

DIA has been accredited as an Authorized Provider by the International Association for Continuing Education and Training (IACET). As an IACET Authorized Provider, DIA offers CEUs for its programs that qualify under the ANSI/IACET Standard. DIA is authorized by IACET to offer 1.0 CEUs for the program. Participants must attend the entire conference 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 conference, sign in at the DIA registration desk each day, and complete the online credit request process through My Transcript. 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 **Tuesday, December 20, 2016.**

View DIA's Grievance Policy at DIAglobal.org/Grievance

ACCESS MY TRANSCRIPT

- Visit DIAglobal.org, select "Sign in" and you will be prompted for your user ID and password
- Choose MENU, found in the upper left corner
- Under CONFERENCES select "Continuing Education"
- Select the blue "My Transcript" button followed by "Credit Request" to process your credit request for the course

ACCESS PRESENTATIONS

- Visit DIAglobal.org
- Select 'Sign in' at the top right
- Enter your User ID and Password
- View 'My Presentation'

Please Note: DIA User ID and Password are needed to access presentations. If you have forgotten your DIA User ID and Password, or this is your first time logging into the DIA website, please use our Login Reminder.

It is DIA policy that anyone in a position to control the content of a continuing education activity must disclose to the program audience (1) any relevant financial relationships related to the content of their presentation and/or the educational activity, and (2) discussions of unlabeled or unapproved uses of drugs or medical devices. Disclosures will be included in the handout materials.

MONDAY, DECEMBER 5

12:00-5:30PM

Registration

1:00-1:25PM

Welcome and Opening Remarks: Setting the Stage

Sudip S. Parikh, PhD
Senior Vice President and
Managing Director
DIA Americas

Chad Gwaltney, PhD
Principal Consultant
Gwaltney Consulting

1:25-2:40PM

Session 1: Keynote Addresses: Perspectives on Study Endpoints: Where Are We Now and Where Are We Going?

Session Chair
J. Jason Lundy, PhD
Principal
Outcometrix

Regulatory Perspective

Janet Woodcock, MD
Director
Center for Drug Evaluation and Research
FDA

Payer Perspective

Mark McClellan, MD, PhD
Director, Duke Robert J. Margolis,
Center for Health Policy
Duke University

Panel Discussion

Study endpoints play a central role in the evaluation of the safety and efficacy of new drugs. For regulatory approval, the selection of an endpoint should represent a clinically meaningful benefit as a direct measure of how a patient feels, functions, and survives. In addition, the selection and evaluation of endpoints is important for health care reimbursement, as well as informing patients and clinicians about the benefits and risks of therapeutic interventions. The Keynote Addresses will discuss the role and evolution of study endpoints in the current environment, and the future of study endpoints in a changing landscape of therapeutic advances and reimbursement schemes.

2:40-3:00PM

Refreshment and Networking Break

MONDAY, DECEMBER 5

3:00-4:30PM

Session 2: Approval Pathways and Endpoint Selection

Session Co-Chairs
Ashley F. Slagle, PhD
Principal, Scientific and
Regulatory Consultant
Aspen Consulting, LLC

Stephen Joel Coons, PhD
Executive Director, PRO Consortium
Critical Path Institute

Selecting and specifying study endpoints in clinical trials can present challenges to drug developers. Different approval pathways, including traditional approval and expedited pathways, as well as the various types of endpoint approaches that are available can expedite drug development, but can also cause confusion and difficulty in decision-making. FDA and Industry speakers will share the history, overview of approval pathways, endpoint examples, decision-making considerations, and case studies to help inform future endpoint decisions for drug development programs.

Overview of Approval Pathways and Implications on Endpoint Selection

Paul Kluetz, MD
Associate Director, Office of Hematology
and Oncology
CDER, FDA

Endpoints Options and Considerations for Selection

Laura Lee Johnson, PhD
Associate Director, Office of Biostatistics,
DB III, Office of Translational Sciences
CDER, FDA

4:30-5:30PM

Session 3: Research Presentations

Session Co-Chairs
René Allard, PhD
Public Disclosure Lead
Grünenthal GmbH, Germany

Jean Paty, PhD
Senior Director and Practice Lead,
Endpoint Strategy
Quintiles

This session will highlight the scientific contributions of new and upcoming investigators involved in study endpoint development, application, and analysis.

Predictors of TMD Persistence: Bringing Science into the Clinic

Carolina Beraldo Meloto, PhD, DDS
Human Pain Genetics Lab,
Faculty of Dentistry
McGill University, Canada

Delivering COA Strategies: Improving Our Understanding of the Patient Experience via High Quality Data

Katherine Zarzar
Manager, Outcomes Measurement
Genentech, A Member of the Roche Group

5:30-6:30PM

Networking Reception

TUESDAY, DECEMBER 6

7:30AM-5:00PM — **Registration**

7:15-8:15AM — **Continental Breakfast and Networking**

8:15-8:30AM — **Welcome to Day Two**

Chad Gwaltney, PhD
Principal Consultant
Gwaltney Consulting

8:30-9:30AM — **Session 4: DIA-ISPOR Session: Prospectively Planning Adaptive Study Designs and Involving All Stakeholders**

Session Co-Chairs
René Allard, PhD
Public Disclosure Lead
Grünenthal GmbH, Germany

Richard J. Willke, PhD
Chief Science Officer
International Society for Pharmacoeconomics and Outcomes Research (ISPOR)

The FDA and EMA are encouraging clinical trials with adaptive features that may make studies more efficient. The IQWiG and other European Health Technology-bodies have been involved in a pilot project with the EMA to share scientific advice on clinical trials. One of the opportunities in the EU brings together HTA stakeholders to share early information about their clinical development plan to establish a consolidated view on trial designs and endpoints. This session will focus on the views of national regulators and payers with regards to their assessment. Can cooperative projects such as the EUROPAIN consortium, which studied the physiology of chronic pain, both from preclinical and clinical mechanistic perspectives, give insights on how adaptive designs can be best planned?

Regulatory Aspects of Adaptive Study Designs

Virtual Presentation

Norbert Benda, PhD
Head of Biostatistics and Special Pharmacokinetics
BfArM, Germany

Accelerated Regulatory Approval What is Needed to Convince the HTA-Body (Case Example)

Virtual Presentation

François Meyer, MD
HAS, Advisor to the President
HAS Haute Autorite De Sante, France

Payer Issues with Adaptive Clinical Trial Designs

Edmund J. Pezalla, MD, MPH
Consultant
EJ Pezalla Independent Consultant

Driving Innovation by Encouraging Cooperative Interactions

Märta Segerdahl, MD, PhD
Chief Medical Specialist, CRD Neurology
H. Lundbeck A/S, Belgium

Panel Discussion

9:30-10:00AM — **Refreshment and Networking Break**

TUESDAY, DECEMBER 6

10:00-11:30AM

Session 5: Addressing Multiple Stakeholder Needs with COAs and Endpoints Selection

Session Chair
Michael Lees
Group Director, WWHEOR Markets (Oncology)
Bristol-Myers Squibb, United Kingdom

Explore the needs of additional stakeholders when selecting endpoints for inclusion in clinical trials and observational research. Health technology assessment (HTA) agencies often have requirements for different endpoints when making decisions, and will often interpret the same endpoint in different ways. Speakers will identify the endpoints required for HTA decision-making, discuss how these differ from regulatory requirements, and explain how the incorrect choice of endpoint, patient population, or study follow-up can cause delays in patient access to medicines. Different perspectives will be provided and potential for greater future alignment between regulators and HTAs - and between different HTAs - will be discussed.

Choosing Endpoints (and Other Key Study Aspects) in Clinical Development: What Does HTA Require in Germany?

Michael Lees
Group Director, WWHEOR Markets (Oncology)
Bristol-Myers Squibb, United Kingdom

Choosing Endpoints (and Other Key Study Aspects) in Clinical Development: What Does HTA Require in the UK?

Pall Jonsson, PhD, MS
Senior Scientific Advisor
National Institute for Health and Care Excellence (NICE), United Kingdom

Academic Perspective: How Can the Choice of Endpoints Targeted at HTAs Help the Assessment of Value?

Lynn D. Disney, PhD, JD, MPH
Director of Research, PATIENTS Program,
Pharmaceutical Health Services Research (PHSR) Department
University of Maryland

11:30AM-1:00PM

Luncheon

1:00-2:30PM

Session 6: Meaningful Score Changes to Establish Endpoint Definitions

Session Chair
David H. Schubert
Vice President of Regulatory and Quality
Stealth BioTherapeutics

Working from the starting point that the concepts we are measuring are meaningful, and that the instruments for measuring these have been developed according to industry standards and have demonstrated measurement properties, it is next necessary to determine what is a meaningful score change in order to establish endpoint specification prospectively. This interactive session will integrate audience status and opinion feedback via their Smartphones.

Performance-Based Measures

Dragos Roman, MD
Team Leader; Division of Metabolism and Endocrinology Products
CDER, FDA

Patient Reported Outcomes Status Update: Challenges, Observations, and (Some) Solutions from the Field

Alan Shields, PhD
Vice President, Patient Centered Outcomes
Adelphi Values

A Novel Method for Estimating Responder Thresholds

Karon Cook, PhD
Research Professor
Northwestern University

2:30-3:00PM

Refreshment and Networking Break

TUESDAY, DECEMBER 6

3:00-4:30PM

Session 7: Clinical Trial Grade Wearables – The Current State of the Science

This session will be a moderated discussion, including your questions and participation on the valid use of wearables in clinical trials, focusing on identification of valid devices and validation of clinical endpoints derived from wearable data.

Session Co-Chairs

Keith W. Wenzel
Senior Director
Perceptive Partner Program

Bill Byrom, PhD
Senior Director, Product Innovation,
Vice Director of ePRO Consortium
ICON plc, United Kingdom

Moderator

Chad Gwaltney, PhD
Principal Consultant
Gwaltney Consulting

Panelists

Elektra J. Papadopoulos, MD, MPH
Acting Associate Director,
Clinical Outcome Assessments Staff
(formerly SEALD), Office of New Drugs, IO
CDER, FDA

Robert A. Vigersky, MD, FACP
Medical Director
Medtronic Diabetes

Robert DiCicco, PharmD
Vice President, Clinical Innovation
and Digital Platforms
GlaxoSmithKline

Keith W. Wenzel
Senior Director
Perceptive Partner Program

Bill Byrom, PhD
Senior Director, Product Innovation,
Vice Director of ePRO Consortium
ICON plc, United Kingdom

4:30-5:00PM

Closing Summary

Session Chair

Chad Gwaltney, PhD
Principal Consultant
Gwaltney Consulting

Panelists

René Allard, PhD
Public Disclosure Lead
Grünenthal GmbH, Germany

J. Jason Lundy, PhD
Principal
Outcometrix

David H. Schubert
Vice President of Regulatory and Quality
Stealth BioTherapeutics

Ashley F. Slagle, PhD
Principal, Scientific and Regulatory Consultant
Aspen Consulting, LLC

Keith W. Wenzel
Senior Director
Perceptive Partner Program

Elektra J. Papadopoulos, MD, MPH
Acting Associate Director, COA Staff (formerly SEALD),
Office of New Drugs, IO
CDER, FDA

5:00PM

Conference Adjourned

DIA

Regulatory Submissions, Information, and Document Management Forum

February 6-8
North Bethesda, MD

One forum, three tracks! Get the latest updates on the emerging operational standards, best practices, and the processes for the submission, creation, and maximum use of regulatory information.

Featured topics:

- RIM for Businesses
- RIM Management for Technology
- Identification of Medicinal Products
- Structured Authoring
- eTrial Master File

Visit DIAglobal.org/RSIDM17

Regulatory Submissions Primer
February 5

Short Courses
February 6

Forum
February 6-8

#RSIDM17

Register and Save $150