DIA

Advancing the Science of Study Endpoints

September 20-21 | Bethesda North Marriott Hotel and Conference Center
North Bethesda, MD

PROGRAM COMMITTEE

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PAREXEL

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. The 2018 Conference will 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. Hear from regulators, payers, patients, measurement experts, and others, as we navigate the complexities of developing and employing appropriate patient-focused endpoints for use in clinical trials. Discussions will cover the use of exit interviews, considerations for time to deterioration endpoints, and navigating the challenges of endpoints in small samples with heterogeneity, among other important topics that are critical across a broad set of stakeholders.

Developed in collaboration with the DIA Study Endpoints Community.

Highlights

- C-Path PRO Consortium update
- Networking reception
- Global regulatory updates
- DIA Study Endpoints Community workstream
- Closing Keynote Address

Who Should Attend

Professionals involved in:

- Industry
- Academia
- Government
- Statistics
- Clinical Trials
- Health Technology involved in:
  - Setting, executing, or evaluating endpoint strategy for drug approval, labeling, promotion, translational science, and market access

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Connect to the Complimentary Conference Wi-Fi
Network: Marriott_Conference
Password: DIA2018

Schedule At-A-Glance

DAY ONE | THURSDAY, SEPTEMBER 20		ROOM
7:00AM-5:30PM	Registration	White Flint Foyer (Lower Level)
7:00-8:00AM	Networking Breakfast	Brookside AB
8:00-8:15AM	**Welcome and Opening Remarks**	White Flint
8:15-9:15AM	**Session 1:** Current Payer Perspective on the Value of Study Endpoints	White Flint
9:15-10:45AM	**Session 2:** How Does "Patient Centricity" and Endpoint Evaluation Align or Differ?	White Flint
10:45-11:15AM	Refreshment and Networking Break	White Flint Foyer
11:15AM-12:15PM	**Session 3:** When Does a Patient Feel Well and How is this Measured	White Flint
12:15-1:15PM	Networking Luncheon and C-Path PRO Consortium Update	Brookside AB
1:15-2:00PM	**Session 4:** Regulatory Update	White Flint
2:00-3:30PM	**Session 5:** Endpoint Evolution During Drug Development – Exit Interviews to Develop Qualitative Evidence for PRO Assessments and Endpoints	White Flint
3:30-4:00PM	Refreshment and Networking Break	White Flint Foyer
4:00-5:30PM	**Session 6:** Endpoint Evolution During Drug Development: Time to Deterioration Endpoint Case Study	White Flint
5:30-6:30PM	Networking Reception	Brookside AB

DAY TWO | FRIDAY, SEPTEMBER 21		ROOM
7:00AM-12:00PM	Registration	White Flint Foyer
7:00-8:00AM	Networking Breakfast	Brookside AB
8:00-8:05AM	**Welcoming Remarks**	White Flint
8:05-9:00AM	**Session 7:** DIA Study Endpoints Community Workstream Update	White Flint
9:00-10:00AM	**Session 8:** The Emerging Role of Novel Devices in Collecting Real Time Data	White Flint
10:00-10:15AM	Refreshment and Networking Break	White Flint Foyer
10:15-11:30AM	**Session 9:** Selecting COA Endpoints and Analyzing COA Data in Rare Disease – Dealing with Small Sample Sizes and Heterogeneity	White Flint
11:30AM-12:30PM	Keynote Closing Address	White Flint
12:30-12:45PM	Closing Remarks	White Flint
12:45PM	**Conference Adjourns**	

Learning Objectives

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

- 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 Credits

DIA is accredited by the Accreditation Council for Pharmacy Education as a provider of continuing pharmacy education. This session is designated for up to 11.75 contact hours or 1.175 continuing education units (CEU's).

ACPE CREDIT REQUESTS MUST BE SUBMITTED BY NOVEMBER 4, 2018

Participants are able to receive an ACPE statement of credit for daily attendance. **No partial daily credit will be awarded.**

Type of Activity: Knowledge

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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 1.2 CEUs for this conference. Participants must complete 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 for the day(s) that you attended the conference, you must sign in each day at the DIA registration desk upon arrival, 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 beginning Thursday, October 4. To view DIA's Grievance Policy, visit DIAglobal.org/CE

Continuing Education Credit Allocation

Day One: Pharmacy 7.25 contact hours or .725 CEUs, UAN: 0286-0000-18-066-L04-P

Day Two: Pharmacy 4.5 contact hours or .45 CEUs, UAN: 0286-0000-18-067-L04-P

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DAY ONE | THURSDAY, SEPTEMBER 20

7:00AM-5:30PM — **Registration**

7:00-8:00AM — **Networking Breakfast**

8:00-8:15AM — **Welcome and Opening Remarks**

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

Emuella Flood, Director, Patient-Reported Outcomes, AstraZeneca

8:15-9:15AM — **Session 1:** Current Payer Perspective on the Value of Study Endpoints

Session Chair
Michael Lees, Head of Value, Evidence and Portfolio Strategy – EUCAN, Takeda Pharmaceuticals International GmbH, Switzerland

This session will describe, from the perspective of 2-3 international payers, the value of study endpoints in making decisions on access to medicines. While highlighting differences between the needs of different jurisdictions and the different emphasis placed upon study endpoints in these jurisdictions, there will be an attempt to find similarities between the different jurisdictions. The session will close with a discussion on likely future requirements for study endpoints from each of the payers included.

Elise Berliner, PhD, Director, Technology Assessment Program, Agency for Healthcare Research and Quality
Donna Messner, PhD, President, Center for Medical Technology Policy (CMTP)

9:15-10:45AM — **Session 2:** How Does "Patient Centricity" and Endpoint Evaluation Align or Differ?

Session Chair
Michael Lees, Head of Value, Evidence and Portfolio Strategy – EUCAN, Takeda Pharmaceuticals International GmbH, Switzerland

This session will describe, from the perspective of patient representatives and endpoint experts, the definition of study endpoints and a common understanding of patient centricity when applied to study endpoints. This common understanding of different perspectives will then be used to discuss and agree on how the development of study endpoints can truly take a patient-centric approach – and what this means for decision-makers. The session will close with a discussion on likely future requirements for study endpoints from each of the payers included.

Phil Posner, PhD, Patient Reviewer/PEAP, Patient-Centered Outcomes Research Institute (PCORI)
Cynthia Grossman, PhD, Director, Science of Patient Input, FasterCures, a center of the Milken Institute
Chad Gwaltney, PhD, President, Gwaltney Consulting

10:45-11:15AM — **Refreshment and Networking Break**

11:15AM-12:15PM — **Session 3:** When Does a Patient Feel Well and How is this Measured

Session Chair
René Allard, PhD, Public Disclosure Lead, Grünenthal GmbH, Germany

The clinical studies section of a products labeling must discuss those clinical studies that facilitate an understanding of how to use the drug safely and effectively. Moreover, the characteristics that are important for understanding how to interpret and apply the study results of the study population should be described. In the 2018 "World Happiness Report", three emerging health problems that threaten happiness were identified by Gallup. The collection of "Quality of Life" data has become routine in clinical trials. Would a "Johari window" type risk approach be pertinent to analyze endpoint objectives in clinical trials and real world evidence collection? Should positive emotional behavioral aspects be introduced to better understand endpoints as being part of a holistic outcome approach?

Acacia Parks, PhD, Chief Scientist, Happify Health
Joseph Pergolizzi, MD, Co-Founder and Chief Operating Officer, NEMA Research, Inc.

12:15-1:15PM

Networking Luncheon and C-Path PRO Consortium Update

Stephen Joel Coons, PhD, Executive Director, PRO Consortium, Critical Path Institute
Sonya Eremenco, MA, Associate Director, PRO Consortium, Critical Path Institute

1:15-2:00PM

Session 4: Regulatory Update

Session Chair
Ashley Slagle, PhD, Principal, Scientific and Regulatory Consulting, Aspen Consulting, LLC

Hear timely updates from FDA representatives on the 21st Century Cures Act provision to expand the qualification and use of drug development tools (DDTs) to support the drug development process. Attendees will hear an update on FDA's patient-focused drug development, including their thoughts on leveraging COAs in cancer clinical trials.

Paul Kluetz, MD, Associate Director for Patient Outcomes (Acting, OCE), FDA
Elektra Papadopoulos, MD, MPH, Associate Director for the Clinical Outcome Assessments (COA) Staff in the Office of New Drugs, CDER, FDA

2:00-3:30PM

Session 5: Endpoint Evolution During Drug Development – Exit Interviews to Develop Qualitative Evidence for PRO Assessments and Endpoints

Session Chair
Ashley Slagle, PhD, Principal, Scientific and Regulatory Consulting, Aspen Consulting, LLC

Exit interviews of clinical trial participants are becoming increasingly common. There are a number of goals and objectives that might be met using these types of interviews. One important focus of these interviews is to develop qualitative evidence to support PRO assessments and endpoints in trials, however, there are no standard methods to guide this type of work or how the data might be used. This session is intended to introduce you to the benefits, considerations, and challenges of using exit interviews to generate qualitative data to develop or support PRO assessments and endpoints. Scientific, operational, and regulatory perspectives will be shared. An overview of the types of goals and objectives that can be achieved using exit interviews, as well as appropriate timing across a drug development program will be discussed. Good methods for exit interviews and suggestions for considering and overcoming operational challenges will be provided. Formal presentations will be followed by a panel discussion and audience questions and answers to ensure important issues surrounding this approach to interviews are covered.

A Regulatory Perspective on Use of Exit Interviews in Clinical Trials to Generate Qualitative Evidence for PRO Assessments and Endpoints
Elektra Papadopoulos, MD, MPH, Associate Director for the Clinical Outcome Assessments (COA) Staff in the Office of New Drugs, CDER, FDA

Qualitative Evidence Generation Using Exit Interviews in Clinical Trials – Good Practices
Cheryl Coon, PhD, Principal, Outcometrix

Exit interviews: Benefits, Challenges, and Operational Considerations
Robyn Carson, MPH, Head, Executive Director of Patient-Centered Outcomes Research, Global Evidence and Value Development, Allergan

3:30-4:00PM

Refreshment and Networking Break

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4:00-5:30PM

Session 6: Endpoint Evolution During Drug Development – Time to Deterioration Endpoint Case Study

Session Chair
Jason Lundy, PhD, Principal, Outcometrix

To sufficiently define a clinical trial endpoint, both a relevant clinical outcome and an appropriate statistical test are needed. In addition, the interpretation (or clinical meaningfulness) of that data also should be specified. This session will describe the distinction between outcomes and endpoints, and discuss the evolution of endpoints throughout the drug development lifecycle. Time to deterioration endpoints in oncology clinical trials will be used as a case-study to illustrate endpoint positioning and interpretation.

From Outcomes to Endpoints
Stephen Joel Coons, PhD, Executive Director, PRO Consortium, Critical Path Institute

Endpoint Selection and Positioning
Lisa Kammerman, PhD, MS, Principal, Kammerman Consulting, LLC

Time to Deterioration Endpoints in Oncology Clinical Trials
Paul Kluetz, MD, Associate Director for Patient Outcomes (Acting), OCE, FDA

Jessica Roydhouse, PhD, ORISE Fellow, OHOP, FDA

Bellinda King-Kallimanis, PhD, Senior Staff Fellow, Social Scientist, OHOP, FDA

5:30-6:30PM

Networking Reception

DAY TWO | FRIDAY, SEPTEMBER 21

7:00AM-12:00PM

Registration

7:00-8:00AM

Networking Breakfast

8:00-8:05AM

Welcoming Remarks

Matthew Reaney, Head of Clinical Outcomes, Sanofi, United Kingdom

8:05-9:00AM

Session 7: DIA Study Endpoints Community Workstream Update

Session Chair
Matthew Reaney, Head of Clinical Outcomes, Sanofi, United Kingdom

This session will provide and introduction to the DIA Study Endpoints Community. Communitiy members will summarize completed and ongoing DIA Study Endpoints workstreams. Additional suggestions for workstreams will be elicited.

The DIA SEC Benefit-Risk Workstream
Adam Gater, MSc, Director, Patient-Centered Outcomes, Adelphi Values

The DIA SEC Wearables Workstream
Emuella Flood, Director, Patient-Reported Outcomes, AstraZeneca

The DIA SEC Endpoint Library Workstream
Matthew Reaney, Head of Clinical Outcomes, Sanofi, United Kingdom

9:00-10:00AM

Session 8: The Emerging Role of Novel Devices in Collecting Real Time Data

Session Chair
Keith Wenzel, Senior Director, Solution Incubator, PAREXEL

Within clinical trials, there is optimism that connected medical devices can be used to collect novel endpoints, to give greater insight into a study subject's health status, to provide benefits of investigational products, and to further the understanding of the safety and efficacy of investigational products. There is also some hesitancy around connected sensors, however, due to the lack of formal guidance from regulatory authorities. The challenge today is not deciding on whether to use a mobile health device, but rather, the effective integration of the device into the study. This session will explore the current state of the science for connected medical devices to collect meaningful study endpoints in clinical trials.

Exploration of Six Sensing Devices as Study Endpoints
Michael King, PhD, MHSc, Senior Director, Diabetes Project Leader, Sanofi

A Regulator's Perspective on Novel Endpoints and Patient Preferences in Medical Devices
Martin Ho, MS, Associate Director of Quantitative Innovation, CDRH, FDA

10:00-10:15AM — **Refreshment and Networking Break**

10:15-11:30AM — **Session 9:** Selecting COA Endpoints and Analyzing COA Data in Rare Disease – Dealing with Small Sample Sizes and Heterogeneity

Session Chair
Emuella Flood, Director, Patient-Reported Outcomes, AstraZeneca

Rare disease populations are typically small and heterogeneous, and often involve different subtypes, such as specific gene mutation subgroups, further reducing sample sizes. Specific COA-related challenges related to small sample sizes and heterogeneity of rare diseases include the selection and validation of COA measures and analyzing and interpreting the COA data to support treatment benefit. This session will identify the challenges of selecting COA endpoints and analyzing COA data in rare disease populations, including in evaluating psychometric properties of COA instruments. Potential solutions will be offered, illustrated though case examples. The regulatory perspective on addressing these challenges will also be presented.

R.J. Wirth, PhD, President and Managing Director, Vector Psychometric Group, LLC

Linda Nelsen, MHS, Senior Director and Head, Value Evidence and Outcomes-Patient Centered Outcomes, GlaxoSmithKline

Jeffrey Palmer, Group Head, Rare Disease Statistics, Pfizer, Inc

Laura Lee Johnson, PhD, Director (Acting), Division III, Office of Biostatistics, Office of Translational Sciences, CDER, FDA

11:30AM-12:30PM — **Keynote Closing Address**

Keynote Speaker
Theresa Mullin, PhD, Associate Director for Strategic Initiatives, CDER, FDA

12:30-12:45PM — **Closing Remarks**

12:45PM — **Conference Adjourns**