DIA

Real World Evidence Conference

Short Course: November 6 | Conference November 9-10 | Virtual

PROGRAM CHAIR

Nancy Dreyer, PhD, MPH, FISPE, DIA Fellow
Chief Scientific Officer and Senior Vice President, Head, Center for Advanced Evidence Generation
IQVIA

PROGRAM COMMITTEE

Steven Anderson, PhD
Director, Office of Biostatistics and Epidemiology, CBER
FDA

Dorothee Bartels, MSc
Head of Global Real World Evidence
UCB Pharma

Brian Bradbury, PhD, MA
Vice President, Center for Observational Research
Amgen, Inc.

Paul Coplan, MBA, FISPE
Vice President, Medical Device Epidemiology & Real-World Data Analytics
Johnson & Johnson

Marni Hall, PhD, MPH
Vice President, Clinical Evidence
IQVIA

James Harnett, PharmD, MS
Executive Director, Health Economics and Outcomes Research
Regeneron Pharmaceuticals, Inc.

Jingyu (Julia) Luan, PhD
Regulatory Affairs Director, BioPharmaceuticals R&D, Late-Stage Development, CRM
AstraZeneca

David Martin, MD, MPH
Vice President, Pharmacovigilance
Moderna

Robert Reynolds
Vice President, Epidemiology & Patient-Centered Outcomes
GlaxoSmithKline

Debra Schaumberg, ScD, OD, MPH
Vice President, Scientific Affairs Real World Evidence
Evidera

Mark Stewart, PhD
Vice President, Science Policy
Friends of Cancer Research

Overview

Real world evidence (RWE) is increasingly becoming important for regulatory decision-making and beginning to touch all areas of the healthcare value chain. Historically used for post-market safety monitoring, sponsors are now beginning to use RWE to support clinical trial design and observational studies in order to generate better treatment approaches, while healthcare systems are collecting and using RWE to substantiate coverage decisions. DIA's *Real World Evidence Conference* will explore new and innovative applications of RWE, and deliver cutting-edge insights in how stakeholders are leveraging RWE to advance healthcare knowledge and decision-making.

Highlights

Short Course on November 6: Introduction to Real World Data for Data Geeks

Who Should Attend

Professionals involved in:

- Real World Evidence
- Epidemiology
- Policy
- Regulatory Science
- Technology development
- Data analytics
- Clinical Research

Schedule At-A-Glance

SHORT COURSE | FRIDAY NOVEMBER 6

12:30-4:30PM	**Short Course:** Introduction to Real World Data for Data Geeks

DAY ONE | MONDAY NOVEMBER 9

10:00-10:15AM	**Welcome and Opening Remarks**
10:15-11:45AM `	**Session 1:** International Updates on RWE in Regulatory Decision-Making
11:45AM-12:15PM	Break
11:45AM-12:15PM	**Exhibitor Event/Non-CE: Case Study Spotlight**
12:15-1:30PM	**Session 2:** The Selection and Use of External Comparators for Expedited Drug Development
1:30-2:00PM	Break
2:00-3:15PM	**Session 3:** Novel Randomized RWE Trial Designs to Inform Regulatory Decisions
3:15-3:45PM	Break
3:45-5:00PM	**Session 4:** Digital Technology in RWD Collection and Analysis
5:15PM-6:00PM	**Exhibitor Event/Non-CE: Happy Hour**

DAY TWO | TUESDAY NOVEMBER 10

9:30AM-10:00AM	**Exhibitor Event/Non-CE: Coffee Corner**
10:00-11:15AM	**Session 5:** What Will the Generation and Application of RWE Look Like in 2030?
11:15-11:45AM	Break
11:45AM-1:30PM	**Session 6:** COVID-19 Hot Topic: Rapid Responses of the RWE Community
1:30-2:00PM	Break
2:00-3:15PM	**Session 7:** Case Study: COVID R&D Alliance, RWE Informing COVID-19 Drug Development
3:15-3:30PM	**Closing Remarks**
3:30PM	Conference Adjourns

Learning Objectives

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

- Explain how RWE is being used today to inform biopharmaceutic development across product lifecycle
- Describe the recent FDA strategic framework for RWD in regulatory decisions
- Evaluate the future applications of RWE in drug development
- Appraise how mobile technologies, AI, machine learning, and other technologies are being used to generate RWE
- Evaluate how patient reported outcomes, EHR, and other patient data is expanding the resources for RWE
- Discuss "lessons learned" from current uses of RWE, and how these can be applied for other future applications of RWE

Continuing Education

Drug Information Association (DIA) is accredited by the Accreditation Council for Pharmacy Education as a provider of continuing pharmacy education. This session is designated for up to 13.25 contact hours or 1.325 continuing education units (CEU's). Type of Activity: Knowledge.

ACPE CREDIT REQUESTS MUST BE SUBMITTED BY THURSDAY, DECEMBER 24, 2020

*ACPE credit is available if you attend the Real World Evidence Conference live November 9-10, 2020. Credit will not be awarded for watching the sessions On Demand post-conference.

CE Allocation

November 6 Short Course: Introduction to Real World Data for Geeks: 3.5 contact hours or .35 CEUs Type of Activity: Knowledge, 0286-0000-20-152-L04-P

November 9 Day 1: Real World Evidence Conference – Day 1: 5.25 contact hours or .525 CEUs Type of Activity: Knowledge, 0286-0000-20-153-L04-P

November 10 Day 2: Real World Evidence Conference – Day 2: 4.5 contact hours or .45 CEUs Type of Activity: Knowledge, 0286-0000-20-154-L04-P

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.** If ACPE credit is not requested by **Thursday, December 24, 2020**, the CEU request will not be transmitted through to the CPE Monitor. 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**.

Continuing Education Credit and My Transcript

If you would like to receive a statement of credit for the days you attend the live virtual conference, you must virtually attend (in their entirety) the short course and/or one or both days of the conference, complete and return a CE Verification of Attendance Form (see instructions below), complete the post program evaluation and request CE credit online through My Transcript (see instructions below). 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 **Tuesday, November 24, 2020**.

If you are claiming ACPE credit for this event you must

1. Attend the entire live virtual short course and/or day one or both days of the conference
2. Complete a Verification of Attendance Form
3. Send back to **CE@DIAglobal.org** by **November 17, 2020**
4. Access your DIA account and select My Transcript to claim your ACPE credit, available on **Tuesday, November 24, 2020**

TO ACCESS MY TRANSCRIPT

- Visit DIAglobal.org
- **Sign In** with your DIA User ID and Password
- Select the Welcome Menu in the upper right hand corner (where your name appears)
- Select **My Account** from the menu
- Select **My Transcripts** then **Manage My Transcripts**

ACCESS PRESENTATIONS

- Visit DIAglobal.org
- **Sign In** with your DIA User ID and Password
- Select the Welcome Menu in the upper right hand corner (where your name appears)
- Select **My Account** from the menu
- Choose **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. **Presentations will be available for six months post conference.*

SHORT COURSE | FRIDAY NOVEMBER 6

12:30-4:30PM

Short Course: Introduction to Real World Data for Data Geeks

Instructors

Simon Dagenais, PhD, MSc, Director, Global Center of Excellence, Real World Evidence, Vertex Pharmaceuticals

Cort Hayflinger, MS, President, Hayflinger Analytic Services, LLC

This short course will provide an overview of analyzing common sources of healthcare real world data (RWD) to develop real world evidence (RWE). The intended audience for this short course includes data science professionals from biopharmaceuticals, consulting companies, academia, government, or policy groups who may work with functions such as RWE, health economics and outcomes research, pharmacoepidemiology, clinical development, medical affairs, or commercial analytics. This course will discuss the regulatory framework related to RWD and RWE, describe common sources and types of RWD available to biopharmaceutical companies through licensing agreements (eg, deidentified medical claims) in the US and elsewhere, and review common use cases for RWD within biopharmaceutical companies, including case studies involving analyses of RWD. This course will also discuss common challenges and opportunities when analyzing RWD.

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

- Examine regulatory framework for RWE and RWD in the US
- Identify common types of RWD available through licensing agreements in the US
- Describe common use cases for RWD in biopharmaceutical companies in the US
- Synthesize case studies involving analyses of RWD
- Evaluate challenges and opportunities for data science to facilitate the analysis of RWD

DAY ONE | MONDAY NOVEMBER 9

10:00-10:15AM

Welcome and Opening Remarks

Robin Weinick, PhD, Senior Vice President and Managing Director, Americas and Global Program Officer, DIA

Nancy Dreyer, PhD, MPH, FISPE, Fellow DIA, Chief Scientific Officer and Senior Vice President, Head, Center for Advanced Evidence Generation, IQVIA

10:15-11:45AM

Session 1: International Updates on RWE in Regulatory Decision-Making

Session Co-Chairs

Nancy Dreyer, PhD, MPH, FISPE, **Fellow DIA**, Chief Scientific Officer and Senior Vice President, Head, Center for Advanced Evidence Generation, IQVIA

Debra Schaumberg, ScH, OD, MPH, Vice President, Scientific Affairs Real World Evidence, Evidera

The advent of the global pandemic of COVID-19, and the resulting imperative for rapid evidence generation, has catapulted conversations around the role of RWE in regulatory decision-making, resulting in both answers and more questions about when RWE is fit-for-use; including retractions of RWE-based manuscripts from major medical journals. This session will bring together regulatory leaders from the United States, Europe, and Japan to discuss recent lessons learned about RWE and its application to the regulatory context, and the frameworks being developed to interrogate such evidence and inform when it can be trusted.

Amy Abernethy, MD, PhD, Principal Deputy Commissioner, FDA

Peter Arlett, MD, Head Data Analytics and Methods Task Force, European Medicines Agency, The Netherlands

Yoshiaki Uyama, PhD, Director, Office of Medical Informatics and Epidemiology, Pharmaceuticals and Medical Device Agency (PMDA)

11:45AM-12:15PM **Break**

11:45AM-12:15PM **Exhibitor Event/Non-CE: Case Study Spotlight**

See Page 8 for more information and instructions on how to RSVP!

12:15-1:30PM **Session 2:** The Selection and Use of External Comparators for Expedited Drug Development

Session Co-Chairs
Robert Reynolds, ScD, FISPE, Vice President, Epidemiology & Patient-Centered Outcomes, GlaxoSmithKline

Mark Stewart, PhD, Vice President, Science Policy, Friends of Cancer Research

For certain clinical settings and scenarios, randomized control trials (RCTs) may be unethical or operationally infeasible. The use of real world data/evidence may provide additional context and supplementary evidence to support regulatory decisions and may be warranted due to the rarity of the disease, scarcity of patients, scientific concerns with data interpretability, or ethical considerations. This session will 1) discuss drug development scenarios that may benefit from the use of real world evidence specifically external comparators, also called synthetic, historical or virtual controls, that supplement or augment RCT data; 2) identify perceived barriers to the use and methodological approaches for constructing external comparator arms; and 3) describe use cases and operational considerations.

Innovative Approaches to External Controls; Synthetic Control Arms
Ruthie Davi, PhD, MS, Vice President, Data Science, Acorn AI, a Medidata Company

Design and Analysis Approaches to Maximize the Validity of ECAs
Jeremy Rassen, DrSc, MS, President and Chief Science Officer, Aetion, Inc.

Building Quality and Traceability into Data Curation
Zoe Li, MBA, Director, Life Science, COTA

Using Comparators for Context: Key Points and Case Examples
Christina Mack, PhD, Vice President, Epidemiology and Clinical Evidence, IQVIA

1:30-2:00PM **Break**

2:00-3:15PM **Session 3:** Novel Randomized RWE Trial Designs to Inform Regulatory Decisions

Session Co-Chairs
Jingyu (Julia) Luan, PhD, Regulatory Affairs Director, BioPharmaceuticals R&D, Late-Stage Development, CRM, AstraZeneca

James Harnett, PharmD, MS, Executive Director, Health Economics and Outcomes Research, Regeneron Pharmaceuticals, Inc.

In recent years, regulators around the world encourage the industry to explore different ways to enhance the use of RWE for demonstrating effectiveness in regulatory submissions. Adopting pragmatism into Randomized Clinical Trial (RCT) design is considered a highly promising strategy of utilizing RWE in regulatory decision-making. The recent COVID-19 pandemic has drawn increased attention to novel approaches for generating evidence and will expand our understanding of the role of novel RWE trial designs. In this session, speakers and panelists from regulatory agencies, industry, and academia will discuss the design, implementation, regulatory acceptance, opportunities and challenges of novel RWE trial designs from policy, methodological, operational, and technical perspectives. Real-case studies (Registry-based Randomized Clinical Trial) that have been accepted by multiple Health Authorities will be shared.

A Case Study of Registry-based Randomized Clinical Trial (R-RCT) for Registration Purpose
Charles Lee, MS, MBA, Executive Director, CVRM Regulatory Affairs, AstraZeneca

Randomized Pragmatic Clinical Trials Utilizing RWD: Myths & Realities
Frank Rockhold, PhD, MSc, Professor of Biostatistics, Duke Clinical Research Institute, Duke University Medical Center

Panel Discussion
Bob Temple, Deputy Center Director for Clinical Science, Office of the Center Director, CDER, FDA

Stefan James, MD, Professor of Cardiology, Uppsala University, Sweden

Frank Rockhold, PhD, MSc, Professor of Biostatistics, Duke Clinical Research Institute, Duke University Medical Center

Zhimin Yang, MD, Division Director, Center for Drug Evaluation, National Medical Products Administration (NMPA)

3:15-3:45PM — **Break**

3:45-5:00PM — **Session 4:** Digital Technology and Machine Learning in RWD Collection and Analysis

Session Chair
Paul Coplan, ScD, MBA, Vice President, Medical Device Epidemiology and Real World Data Analytics, Johnson & Johnson

Data gathered through personal digital devices is a rapidly growing area of RWD that can be used to understand patient health and quantify interventions' effects on health in new ways. Such mobile health technologies can be used to aggregate data from wearable technologies, collect data on patient reported outcomes from patient-completed self-assessment questionnaires, and aggregate patient clinical records (eg, EHR and claims data) across multiple healthcare provider systems. In addition, the data generated by wearable technologies from the monitoring of human physical activities and behaviors, as well as physiological and biochemical parameters during daily life, are ripe for the application of pattern-recognition tools such as machine learning and artificial intelligence. The goal of this session is to learn from visionaries in the field about the potential for using digital technology for collecting RWD for health assessment and evaluation, and approaches to analyzing digital data to provide clinically relevant insights.

Incorporating Patient Reported Outcomes and Wearables into Studies: Progress with the MyStudy App at FDA
Kenneth Quinto, MD, MPH, Senior Medical Advisor for Real World Evidence Analytics, OMP, CDER, FDA

Using Digital Apps to Improve RWE Studies: Lessons Learned with Hugo Digital App
Harlan Krumholz, Harold H. Hines, Jr. Prof of Medicine and Director, Center for Outcomes Research, Yale University

When ML Models Can be Helpful vs Standard Regression Models in Prediction Models Using Digital Data of Treatment Safety and Effectiveness
Michael Kattan, PhD, Department Chairman, Quantitative Health Sciences, Cleveland Clinic

5:15PM-6:00PM — **Exhibitor Event/Non-CE: Happy Hour**

See Page 8 for more information and instructions on how to RSVP!

DAY TWO | TUESDAY NOVEMBER 10

9:30AM-10:00AM — **Exhibitor Event/Non-CE: Coffee Corner**

See Page 9 for more information and instructions on how to RSVP!

10:00-11:15AM — **Session 5:** What Will the Generation and Application of RWE Look Like in 2030?

Session Co-Chairs
Dorothee Bartels, MSc, Head of Global Real World Evidence, UCB Pharma

Steve Anderson, PhD, Director, Office of Biostatistics and Epidemiology, CBER

This session will explore what the successful generation and application of RWD and RWE might look like in 2030. It is expected that development of key elements such as robust data, validated methods

and knowledge, as well as engagement of a broad range of interdisciplinary experts including data scientists, epidemiologists, statisticians, clinical scientists will be critical to acceptance of RWE in supporting claims of therapeutic effectiveness. Success may further include surmounting challenges such as integration of high-quality data from a variety of sources, development of new and innovative methods, bridging accross multiple disciplines and stakeholders and others. Panel members will discuss these and other critical elements necessary for the successful generation and application of real world evidence in 2030.

Jon Duke, MD, Director of Health Informatics, Georgia Tech Research Institute

Richard Forshee, PhD, Associate Director for Analytics and Benefit-Risk Assessment, CBER, FDA

11:15-11:45AM — **Break**

11:45AM-1:30PM — **Session 6:** COVID-19 Hot Topics: Rapid Responses of the RWE Community

Session Co-Chairs
Marni Hall, PhD, MPH, Vice President, Clinical Evidence, IQVIA

Richard Forshee, PhD, Associate Director for Analytics and Benefit-Risk Assessment, CBER, FDA

This session provides examples of the role RWE plays to inform the pandemic response. Specific use cases including evaluation of the natural history of the disease, comparative studies of therapeutics, and establishment of a near real-time community reporting system. An emphasis will be placed on lessons learned from the need for rapid collaboration and adaptation.

Speakers
Robert Ball, MD, MPH, MSc, Deputy Director, Office of Surveillance and Epidemiology, CDER, FDA

Patrick Ryan, Head, Epidemiology Analytics, Janssen Pharmaceuticals, Inc.

Jeff Allen, PhD, President and Chief Executive officer, Friends of Cancer Research

Nancy Dreyer, PhD, MPH, FISPE, Fellow DIA, Chief Scientific Officer and Senior Vice President, Head, Center for Advanced Evidence Generation, IQVIA

1:30-2:00PM — **Break**

2:00-3:15PM — **Session 7:** Case Study: COVID R&D Alliance, RWE Informing COVID-19 Drug Development

Session Co-Chairs
Brian Bradbury, PhD, MA, Vice President, Center for Observational Research, Amgen, Inc.

Marni Hall, PhD, MPH, Vice President, Clinical Evidence, IQVIA

This session provides an introduction to the COVID R&D Alliance and how the use of RWE is assisting in the design of platforms trials and providing evidence regarding the natural history of disease for patients diagnosed with COVID-19. Emphasis will be placed on the value of coordinated and collaborative engagement across manufactures and the lessons learned from this effort.

Speakers
Anne Heatherington, Senior Vice President, Head of Data Sciences Institute, Takeda

Cathy Critchlow, Vice President, Center for Observational Research, Amgen

Ying Bao, Senior Director, Center for Observational Research and Data Science, Bristol Myers Squibb

3:15-3:30PM — **Closing Remarks**

Brian Bradbury, PhD, MA, Vice President, Center for Observational Research, Amgen, Inc.

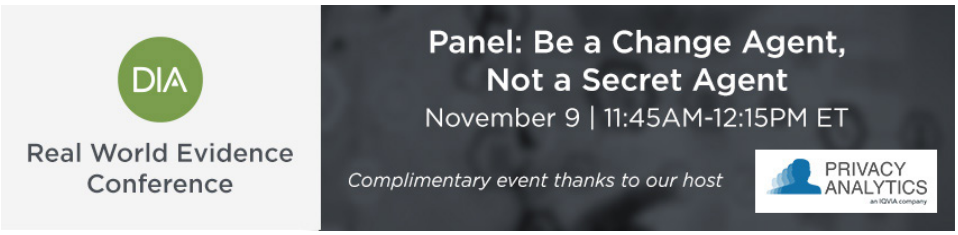
3:30PM — **Conference Adjourns**

Real World Evidence Exhibitor Sponsored Events

Separate RSVP is required for each event. Visit the exhibitor directory for more information

MONDAY | NOVEMBER 9

11:45AM-12:15PM ET **Exhibitor Event/Non-CE:** Case Study Spotlight hosted by Privacy Analytics



Panel: Be a Change Agent, Not a Secret Agent

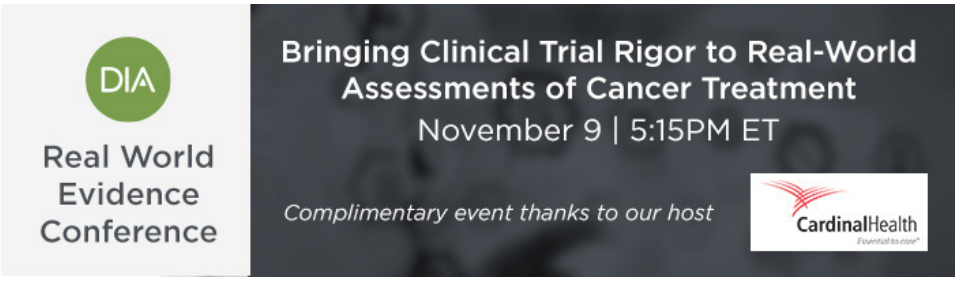
The healthcare industry is rapidly evolving into an ecosystem replete with data-rich, digitally enabled stakeholders. This shift is eroding drug manufacturers' informational advantage within the healthcare value chain. It's forcing sponsors to embrace an operating model anchored on collaboration, openness and trust. One where sponsors play the valued role of change agent - actively sharing sensitive health data with partners to foster greater advancements in patient health. This discussion will dissect today's dynamic business environment and place special emphasis on data platforms as a launchpad for innovation that benefits everyone.

Sarah Lyons, General Manager, Privacy Analytics

Tom Baker, Vice President Consulting, Europe, Middle East, Africa (EMEA), IQVIA

Separate RSVP is required. ***Click here to RSVP!***

5:15PM-6:00PM ET **Exhibitor Event/Non-CE:** Happy Hour hosted by Cardinal Health



Bringing Clinical Trial Rigor to Real-World Assessments of Cancer Treatment

More than 95% of cancer patients are treated in the real world, versus in clinical trial settings. However, drug developers and other industry stakeholders are still in the formative stages when it comes to developing reliable models for generating RWE that are accurate, consistent and approximate the measure used in clinical trials.

Join us for an interactive happy hour exploring RWE generation, and assessing a new methodology that brings clinical trial rigor to real-world data.

BONUS: A $10 Amazon eGift card will be given to those that attend (must be a registered attendee for DIA's Real World Evidence Conference to qualify).

Andrew Klink, PhD, MPH, Lead Scientist, Real-World Evidence and Insights, Cardinal Health

Separate RSVP is required. ***Click here to RSVP!***

9:30AM-10:00AM ET **Exhibitor Event/Non-CE:** Coffee Corner hosted by Indegene Inc.

New Indications Discovery – Leveraging RWD/RWE Insights For Product Optimization

- The session will explain how RWE insights can identify new indications for in-market products and pre-launch candidate drugs.
- Real world examples will be provided to illustrate where this can benefit an organization's portfolio
- How applied use of AI / ML can generate insights into disease progression and prediction of treatment responsiveness in target populations.

BONUS: A $5 Starbucks eGift card will be given to those that attend (must be a registered attendee for DIA's Real World Evidence Conference to qualify).

Reena Gollapudy, PhD, Head of RWD/RWE, Indegene Inc.

Separate RSVP is required. ***Click here to RSVP!***