Renaissance Baltimore Harborplace Hotel

Oct 16, 2023 7:30 AM - Oct 17, 2023 6:30 PM

202 E Pratt Street, Baltimore, MD 21202, USA

Real-World Evidence Conference

Translating Insights into Real-World Value

Print Agenda

Day 1 Oct 12, 2023

10:00 AM — 2:00 PM

Short Course: How Good is Good Enough? Fit-for-Purpose Considerations for RWD/RWE for Regulatory Purposes

Day 2 Oct 13, 2023

10:00 AM — 2:00 PM

Short Course: Measuring the Quality of Real-World Data (RWD)

Day 3 Oct 16, 2023

7:30 AM — 5:30 PM | Maryland Ballroom Foyer Reg

Conference Registration

7:30 AM — 8:30 AM | Baltimore Ballroom

Networking Breakfast

8:30 AM — 8:45 AM | Maryland Ballroom

Opening Remarks

Opening Remarks

Track: General Session

Session Chair(s)

Tamei Elliott, MS

Associate Director, Scientific Programs
DIA, United States

Tamei Elliott, MS, serves as the Associate Director of Scientific Programs for the Americas region at DIA. In this pivotal role, she is responsible for identifying and prioritizing content areas and topics crucial to DIA constituents. Tamei assesses the implications of significant regulatory and health policy changes, seamlessly integrating relevant content into the development and advancement of DIA conferences and courses. Her responsibilities extend to overseeing content development and strategy within the Americas region.

David Martin, MD, MPH

Vice President, Global Head RWE
Moderna, United States

David Martin leads Global RWE for Moderna. Previously he completed 20 years of active duty service split between the United States Air Force and Public Health Service. At the FDA he led the Division of Epidemiology in the Center for Biologics. Subsequently, he established the Real World Evidence group in

the Office of Medical Policy to drive the agency's scientific, guidance, and submission review responses to the RWE provisions of the 21st Century Cures Act. He initiated key RWE pathfinding efforts with external stakeholders including RCT Duplicate and the open-source FDA MyStudies mobile application. He holds an MD and MPH from Johns Hopkins and is board certified in occupational & environmental medicine as well as clinical informatics.

8:45 AM — 10:00 AM Maryland Ballroom

Session 1: A Year in Review

This opening session will provide attendees with an overview of key events related to RWE in the past year (2022-2023). This session will set the stage and introduce various topics that will be discussed in greater detail in other sessions later in the agenda.

To help attendees understand why the key events presented are important to the field of RWE, they will be placed in a historical context to describe how they build on previous events and contribute to the evolution of RWE. This session will focus primarily on the following groups of stakeholders:

1. Biopharmaceutical companies who acquire and analyze RWD to develop RWE
2. Regulatory agencies who review RWE and make regulatory decisions based on RWE
3. Academic researchers who propose analytical methods and standards for the analyses of RWD
4. Private industry vendors who develop, aggregate, disseminate, and analyze RWD

Learning Objective : At the conclusion of this session, participants should be able to:

- Identify key events related to RWE in the past 1 year period
- Cite these events in a historical context to describe how they build on previous key events in RWE
- Examine how these events are interrelated and contribute to the evolution of RWE

Track: General Session

Session Chair(s)

Simon Dagenais, PhD, MSc

Real-World Evidence Lead, Internal Medicine
Pfizer Inc, United States

Simon is an epidemiologist and health economist with expertise in designing, conducting, and communicating scientific studies related to the clinical and economic value of therapies for neurologic conditions. He is currently the global RWE lead for Internal Medicine at Pfizer. Prior to Pfizer, Simon was the global lead for Neurology in the RWE COE at Vertex Pharmaceuticals and supported programs for acute pain, Duchenne muscular dystrophy, and other neurologic conditions. Prior to Vertex, Simon worked in RWE, health economics and outcomes research, and pharmacovigilance at Pacira Pharmaceuticals.

Speaker(s)

EMA Update

Stefanie Prilla, DrSc

RWE Coordinator
European Medicines Agency, Netherlands

Dr. Stefanie Prilla is a pharmacist and doctor of natural sciences. Stefanie has more than 15 years of experience in the field of regulatory science with a focus on EU marketing authorisations. Since 2007, she has been working for the European Medicines Agency (EMA). Before joining the EMA data analytics task force, Stefanie held the post of PRIority MEdicines (PRIME) Scientific Coordinator. Previously, Stefanie supported the set up of the European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP) as well the IMI PROTECT project which was led by EMA. In her current role, Stefanie coordinates EMA studies using real-world data to support EU regulatory decision making.

A Short Trip Around the World: A Simple Understanding of the Complex Web of Regional Regulatory Guidances

Rob Kalesnik-Orszulak, PharmD

Senior Director, Global Regulatory Strategy
Bristol Myers Squibb, United States

Rob Kalesnik-Orszulak is a Senior Director of Global Regulatory Strategy at Bristol Myers Squibb. As Team Lead for genitourinary (GU) tumors, Rob has built a high performing team of global regulatory leads each working on a number of global filings in renal, bladder, and prostate cancer. As the Regulatory Innovation Lead for Real World Evidence (RWE) and Data Science, Rob is also responsible for establishing the regulatory department's expertise, capabilities, and strategy around RWE across all therapeutic areas at BMS. Rob has over 8 years of experience leading regulatory strategy across the drug development lifecycle, from first-in-human trials to mature products, with a focus in oncology.

GetReal Institute: Developing a Multistakeholder Approach to Advance the use of RWE for Better Healthcare Decision-making

Shahid Hanif, PhD, MSc

Managing Director
GetReal Institute, Netherlands

Shahid Hanif is the Managing Director of the GetReal Institute, a not-for-profit multi-stakeholder association based in the Netherlands, which aims to facilitate the adoption and implementation of real-world evidence in regulatory, HTA and clinical decision-making in Europe. He leads the GetReal Institute, which follows two Innovative Medicines Initiative (IMI) funded programmes, to establish it as a leading independent and sustainable European forum for stakeholder dialogue, consensus development and co-creation of solutions to advance the use of real-world evidence.

10:00 AM — 10:45 AM Baltimore Ballroom

Refreshment and Networking Break

10:10 AM — 10:40 AM Homeland

Hosted Event/Non-CE: Case Study Spotlight Hosted by OM1: A New Era of Integrated Evidence Generation

Integrated evidence generation frameworks leverage an ensemble of existing real-world data sources and new data collection to generate evidence that is 'fit-for-purpose' and used for critical decision making across the product life cycle.

Join this session to explore case examples of how OM1's real-world data networks and proprietary automated data collection and processing platforms enable rapid, cost-effective, and scalable integrated evidence generation.

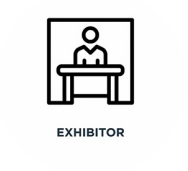
Featured Topics

- Collect longitudinal data and patient outcomes, rapidly and at scale
- Reduce enrollment burden on sites
- Enable additional studies as needed with minimal effort

• Meet multiple stakeholder needs with one platform • Publish results rapidly

Track: Exhibitor Event

Session Chair(s)



Sponsored Sessions

United States

Speaker(s)

Exhibitor

Sonja Wustrack

Managing Director, Evidence Generation Networks
OM1, United States

10:45 AM — 12:00 PM Maryland Ballroom

Session 2: Standardization of Real-World Data for Regulatory Submissions

This session will examine the use of data standards for the formatting and submission of data sets using real-world data. Speakers will address the use of various data standards and will speak to the utility of these various standards to facilitate the analysis and submission of real-world data. A short panel discussion will follow.

Learning Objective : At the conclusion of this session, participants should be able to:

- Recognize the importance of common data models for real-world data
- Identify various data standards used for the analysis and submission of real-world data
- Discuss challenges associated with application of different data standards

Track: General Session

Session Chair(s)

Brad Jordan, PhD

Associate Vice President, Regulatory Policy and Strategy
Eli Lilly and Company, United States

Dr. Brad Jordan is a Head of Regulatory Affairs Policy at Flatiron Health, where his team works to advance the use of Real-World Evidence for regulatory decision-making. Brad was at Amgen for 15 years prior to joining Flatiron, where he led Global Regulatory and R&D Policy for Oncology and for Biosimilars and Biologics.

Speaker(s)

FDA Demonstration Projects

Motiur Rahman, PhD, MPharm, MS

Senior Epidemiologist, Real World Evidence Analytics, OMP, CDER
FDA, United States

Motiur Rahman, MPharm, MS, PhD, is an Epidemiologist at Real-World Evidence (RWE) Analytics in the Office of Medical Policy, CDER, FDA. His responsibilities include developing guidance, improving internal Agency processes, stakeholder engagement, collaborating on Agency-funded demonstration projects, and providing consultancy on RWE study submissions. He joined FDA in April 2022 after working as an epidemiologist in industry settings.

CDISC Standards and the Use of Real-World Data

Bess LeRoy, MPH

Head of Standards Development
CDISC, United States

Bess LeRoy has over 15 years of experience working in public health research. She is currently the Head of Standards Development at CDISC. She has been a CDISC team member since 2011 and has been involved in the development of over 16 CDISC Therapeutic Area User Guides. Bess has a BS from the University of Michigan, an MPH from Boston University, and is currently pursuing a DrPH from Johns Hopkins University.

Know Thy Data: Trusting RWD in Common Data Models for Regulatory Submissions

Mayur Saxena, PhD

Chief Executive Officer
Droice Labs, United States

As an entrepreneur and scientist, Mayur has concentrated on advancing medicine with high-noise, big data analysis. Before founding Droice, he played key roles in several startups, including co-founding a biotechnology firm in the diabetes space. He earned his BTech at IIT Kanpur and his MS and PhD at Columbia University, focusing on the computational physics of disease.

Adapting Real-World Data (RWD) into CDISC Submission Standards: Challenges and Potential Solutions

James Browning, MPH

Director of Biostatistical Programming, Center for Observational Research
Amgen, United States

James Browning, MPH, is a Director of Biostatistical Programming in the Center for Observational Research (CfOR) at Amgen Inc. He has over 14 years of experience conducting observational studies in multiple therapeutic areas with the majority concentrated in oncology. He is skilled in statistical analysis, data visualization and data management with a focus on RWE used for regulatory decision-making. Currently, he leads multiple programming teams focused on generating RWE to support the development and continuous benefit:risk assessment of Amgen's medicines. He also has an interest in the new regulatory guidelines for generating RWE and is exploring processes and standards to align with the evolving framework.

12:00 PM — 1:00 PM Baltimore Ballroom

Networking Luncheon

1:00 PM — 2:15 PM Maryland Ballroom

Session 3: Methodological Insights on External Controls and Sensitivity Analyses

This session will highlight specific methodological issues relevant to real-world evidence. The first presentation will discuss results obtained when applying different methods to analyze data in externally controlled trials; the second presentation will discuss how external control arm data can help interpret results from single-arm trials; the third presentation will discuss a structured framework for sensitivity analyses to assess unmeasured confounding. A panel discussion will follow.

Learning Objective : At the conclusion of this session, participants should be able to:

- Describe various statistical approaches for analyzing data in externally controlled trials
- Identify how external control arm data can be used to inform early decisions in drug development programs
- Recognize statistical methods used in sensitivity analyses to assess unmeasured confounding

Track: General Session

Session Chair(s)

John Concato, MD, MPH, MS

Associate Director for Real-World Evidence Analytics, OMP, CDER
FDA, United States

Dr. John Concato is Associate Director for Real-World Evidence Analytics in the Office of Medical Policy, Center for Drug Evaluation and Research, FDA. As an internist and epidemiologist, his responsibilities related to real-world evidence (RWE) include developing internal Agency processes, interacting with external stakeholders, and coordinating demonstration projects as well as guidance development. Dr. Concato joined FDA from Yale School of Medicine and the U.S. Department of Veterans Affairs, where he was a clinician, educator, independent investigator, research center director, and Professor of Medicine. He has a BE degree from The Cooper Union, MD & MS degrees from New York University, and an MPH degree from Yale University.

Speaker(s)

To Pursue or Not? How External Controls Were Used to Decide the Course of Two New Recurrent Glioblastoma Treatments

Lisa Ensign, PhD, MSc

VP, Data Science
Medidata AI, United States

Lisa Ensign is a VP of Data Science at Medidata AI. Her current work in the Integrated Evidence team is focused on creating analytical approaches to improve and transform the efficiency and rigor of clinical trials, centered on the use of external controls. Dr. Ensign began her career at MD Anderson Cancer Center and has over 30 years experience in the life sciences sector. She received her MS in biostatistics from Harvard University and her PhD in clinical science from the University of Colorado, where she is also an instructor on ethics and the responsible conduct of research.

Deriving External Controls Arms From RWD: G-Computation and Digital Twins Applied to a Small Single Arm Phase 1 Trial

David Paulucci, MSc

Director, Data Science
BMS, United States

David Paulucci is Director of Data Science in the Department of Global Biometrics and Data Sciences at Bristol Myers Squibb. In his role, David is responsible for conducting exploratory analyses, and developing statistical learning models to support global drug development in oncology. Prior to working at BMS, he spent 3 years at Mount Sinai Hospital, carrying out analyses leading to more than 20 publications on surgical outcomes for localized kidney and prostate cancer. David received his MS in Biostatistics from the Icahn School of Medicine at Mount Sinai. His key areas of interest include supervised machine learning, and predictive modeling of heterogenous treatment effects.

Evaluating the Robustness of Real-World Evidence through Quantitative Sensitivity Analysis for Unmeasured Confounding

Mingyang Shan, PhD

Senior Advisor
Eli Lilly and Company, United States

Mingyang Shan is a senior research advisor in the Real-World & Access Analytics Capabilities team at Eli Lilly and Company, where he applies a spectrum of advanced analytic methods to deliver RWE and enable innovative evidence generation. His current focus is on developing methodology and best analytical practices to leverage evidence from real world data in drug development. Prior to joining Eli Lilly, Mingyang completed his Ph.D in biostatistics at Brown University where his research interests converged at the intersection of missing data, causal inference, and Bayesian inference.

2:25 PM — 3:40 PM Maryland Ballroom

Session 4: “Tokenization”: Privacy-Preserving Data Integration to Enhance Clinical Trials and Real-World Evidence Studies

In this session, conference attendees will learn about the methods and procedures underlying the tokenization of healthcare data, hear first-hand experiences from researchers using integrated data to conduct studies, gain perspectives

on the future landscape of interacting with the tokenized world of healthcare data, and participate in a discussion surrounding challenges and opportunities.

Learning Objective :

- Explain the methodological approaches for generating privacy-preserving record linkage using tokenization
- Appraise the value of and challenges in using tokenized healthcare data
- Describe practical applications including the areas of clinical trials and post-authorization safety studies
- Summarize the potential benefits of linking healthcare data using tokenization to multiple stakeholder groups including patients, HCPs, industry sponsors and regulators

Track: General Session

Session Chair(s)

Keri Monda, PhD, MS

Executive Director, Center for Observational Research
Amgen, United States

Keri Monda, PhD, is an Executive Director of Observational Research and Head of the Data & Analytics Center within the Center for Observational Research (CfOR) at Amgen. In her role, she leads a team of epidemiologists and data scientists responsible for generating real-world evidence in support of programs from research and early development through launch and end of patent expiry, and oversees a large, integrated real-world data and analytics ecosystem. Prior to her time at Amgen, Keri was a genetic epidemiologists on faculty in the Department of Epidemiology at the University of North Carolina, Chapel Hill.

Daina Esposito, PhD, MPH

Executive Director, Global Safety Epidemiology
Moderna, United States

I shape and execute quantitative strategies for safety signal identification and evaluation, assessment of risk-benefit balance, and conduct of real-world evidence studies as lead for the Global Safety Epidemiology team at Moderna. In this capacity, I work closely with a cross-functional team of physicians, pharmacovigilance scientists, biostatisticians, and experts in regulatory science to ensure comprehensive assessment of the safety profile of Moderna's products.

Speaker(s)

Nuts and Bolts of Data Tokenization and Linkage

Andrew Kress

Chief Executive Officer
HealthVerity, Inc., United States

Andrew is the CEO of HealthVerity, a company that operates a healthcare data marketplace using novel technologies to connect data at the patient level from a broad set of external data partners. Previously, he was SVP, Healthcare Value Solutions for IMS Health, responsible for real world evidence solutions, payer and provider services, and clinical

trial optimization solutions. Prior, he was the CEO of SDI Health, a leader in healthcare data & analytics prior to its acquisition by IMS Health.

Unlocking Insights: Lesson Learned from the "Tokenization" of a Psoriasis Trial

Alexander Liede, PhD, MSc

Senior Director, Global Epidemiology
Abbvie, Ireland

Alex is a Senior Director and Head of Evidence & Partnerships in Global Epidemiology at AbbVie based in Dublin, Ireland. Alex has 20 years of experience in the biopharma industry and leads a team of epidemiologists focused on real-world evidence solutions and innovative approaches using real-world data in support of safety strategy and cross-functional initiatives in drug development. Before joining AbbVie in 2019, Alex worked at Amgen in the Center for Observational Research, as a research scientist at Cedars-Sinai Medical Center in Los Angeles, and as a genetic counsellor with Cancer Care Ontario and Women's College Hospital in Toronto, Canada.

Future of External Data Use in Research: How Do We Get There?

Joseph B. Franklin, JD, PhD

Head of Strategic Affairs
Verily, United States

Joe Franklin leads the Strategic Affairs team at Verily and focuses on evidence generation strategy, including regulatory and privacy strategy at the intersection of clinical research and care. Before joining Verily in 2021, Joe held a variety of positions at FDA, including as senior advisor on data and evidence initiatives. Joe led the biosimilars policy staff in the Office of New Drugs and served as an attorney in the chief counsel's office for multiple periods during his career at FDA, including during COVID-19, when he advised FDA on emergency use authorizations and supported the U.S. government's global vaccine deployment. Joe has a PhD in cell biology from his early career as a bench scientist.

3:40 PM — 4:10 PM Homeland

Hosted Event/Non-CE: Case Study Spotlight hosted by Truveta: Using the EHR to identify Long COVID patients in near-real-time: A feasibility assessment of Truveta Data

Truveta Data includes full medical records for nearly 100 million patients across the United States from more than 31 health systems. As researchers race to better understand Long COVID as an emerging disease, near-real-time EHR data

can be utilized to describe characteristics of this patient population, such as information on demographic factors and social drivers of health, along with differences in vaccination status prior to COVID-19 onset and post-COVID symptomology. Further, Truveta Data allows researchers to quickly explore different data definitions and algorithms for identifying patient with Long COVID as our understanding of this disease evolves.

Track: Exhibitor Event

Session Chair(s)

EXHIBITOR

Sponsored Sessions

United States

Speaker(s)

Michelle Iannacone

Epidemiologist, Methods Lead
Pfizer, United States

Exhibitor

Sally Omidvar

VP, Life Science Research Success
Truveta, United States

Sally Omidvar is the VP of Life Science Research Success at Truveta where she is responsible for enabling and supporting the research of Truveta's life science and medical device customers. Prior to Truveta, Sally was CTO for Healthcare at DataRobot where she worked to develop and expand machine learning use cases among payers, providers, and life science customers, as well as managing large scale COVID-19 engagements with HHS, CDC, and FDA. Prior to DataRobot her work included collaborations with Google and Gavi utilizing artificial intelligence to inform last-mile vaccine logistics and as a researcher at the CDC engaged in international emergency response.

3:40 PM — 4:10 PM Baltimore Ballroom

Refreshment and Networking Break

4:15 PM — 5:30 PM Maryland Ballroom

Session 5: Case Studies from Recent Approvals of RWD/RWE Submissions

Session 5: Case Studies from Recent Approvals of RWD/RWE Submissions

Learning Objective : At the conclusion of this session, participants should be able to:

- Explore FDA and pharmaceutical industry perspectives on the potential role of RWE in regulatory decision-making
- Identify relevant considerations for the use of RWD and RWE to support regulatory decision-making
- Apply lessons learned from recent use cases including submissions containing RWE to support safety and effectiveness

Track: General Session

Session Chair(s)

Yun Lu, PhD, MS

Mathematical Statistician, Office of Biostatistics and Pharmacovigilance, CBER
FDA, United States

Dr. Yun Lu is a Mathematical Statistician and real-world evidence (RWE) reviewer working for the Food and Drug Administration (FDA)/Center for Biologics Evaluation and Research (CBER)/Office of Biostatistics and Pharmacovigilance (OBPV). Dr. Lu received her Ph.D. in Biostatistics from Johns Hopkins Bloomberg School of Public Health. Dr. Lu joined FDA/CBER in 2010 and she has extensive experiences with reviewing RWE related submissions and conducting vaccine safety and effectiveness studies using real-world data (RWD) including Medicare claims data from the Centers for Medicare and Medicaid Services (CMS).

Speaker(s)

CDER RWE Program: Update and Examples

Kimberly Smith, MD, MS

Real-World Evidence Analytics Team, Office of Medical Policy, CDER
FDA, United States

Kimberly Smith is a nephrologist with the Real-World Evidence Analytics team in the Office of Medical Policy (OMP), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration. In her current role, she develops and implements programs and policies related to the use of real-world evidence in drug development. Prior to her current role, Dr. Smith served at FDA as team leader for the Division of Clinical Trial Quality in OMP and as the nephrology team leader in the Division of Cardiology and Nephrology in CDER's Office of New Drugs. Before joining the FDA, she was with the Coverage and Analysis Group at the Centers for Medicare and Medicaid Services.

Oncology RWE Program: Overview and Regulatory Perspective on Effectiveness

Donna Rivera, PharmD, MSc

Associate Director for Pharmacoepidemiology, OCE
FDA, United States

Donna R. Rivera, PharmD., MSc., is the Associate Director of Pharmacoepidemiology in the Oncology Center of Excellence at the US Food and Drug Administration. She leads the Oncology Real World Evidence (RWE) Program, focused on the use of Real World Data (RWD) and RWE for regulatory purposes as well as management of the RWD research portfolio strategy and development of related regulatory policy to support the OCE mission. Dr. Rivera has interests in the use of RWD to increase knowledge of unrepresented populations and advance health equity, observational study designs and RWD methodological approaches, and appropriate uses of RWD for drug development to increase access of effective therapies to patients.

FDA Accelerated Aproval Based on Real-World Evidence

Adrian Cassidy, PhD, MSc

Head Global Evidence Generation
Novartis, Switzerland

Adrian Cassidy is the Vice President and Head of Global Evidence Generation at Novartis with responsibility to develop novel data and evidence applications across the development lifecycle of innovative medicines. As an epidemiologist, Adrian has over 20 years of industry and public sector experience in real world evidence generation, as well as an established track record of building and leading highly successful evidence generation groups and data/digital/analytics platforms. Adrian is passionate about solving healthcare challenges and transforming clinical practice to deliver outcomes that matter for patients.

5:30 PM — 6:30 PM Baltimore Ballroom

Networking Reception sponsored by Merative

Day 4 Oct 17, 2023

7:15 AM — 4:10 PM Maryland Ballroom Foyer Reg

Conference Registration

7:15 AM — 8:00 AM Baltimore Ballroom

Networking Breakfast

7:30 AM — 8:00 AM Homeland

Hosted Event/Non-CE: Case Study Spotlight Sponsored by Clinetic: Accelerating Research Through Electronic Health Record (EHR) Surveillance

The traditional approach to capturing clinical information from medical records is labor-intensive and slow. Chart abstraction for a sampling of cases from a single institution often requires manual effort from nurses, who are increasingly in short supply and burdened with greater clinical workloads. With the increased utilization of Electronic Health Record (EHR) systems, it is now possible to rapidly curate real world clinical data and keep it refreshed continuously ultimately enabling study teams to accelerate clinical research and evidence generation.

Learning Objective : Featured Topics:

- Using timely EHR surveillance to track disease epidemiology and healthcare utilization in near real-time
- Leveraging EHR connectivity and health system relationships to identify and enroll patients into prospective studies
- Applying Natural Language Processing (NLP) to curate clinical concepts from unstructured EHR data

Track: Exhibitor Event

Session Chair(s)

EXHIBITOR

Sponsored Sessions

United States

Speaker(s)

Exhibitor

Tom Kaminski

CEO
Clinetic, United States

Tom is the co-founder and CEO of Clientic, a health technology company harnessing the potential of electronic health record (EHR) data to accelerate clinical research and evidence generation. Previously, he worked at LabCorp for ten years where he led Corporate Strategy and the launch of several new products and business units. Tom also previously worked at Duke University where he led business strategy for the Duke Clinical Research Institute (DCRI) and led the launch of the Duke Institute for Health Innovation (DIHI).

8:00 AM — 9:15 AM Maryland Ballroom

Opening Remarks and Session 6: Objectivity and Transparency: Roundtable Discussions on Real-World Studies to Support Regulatory Decision-making

Opening Remarks and Session 6: Objectivity and Transparency: Roundtable Discussions on Real-World Studies to Support Regulatory Decision-making

Learning Objective : At the conclusion of this session, participants should be able to:

- Discuss opportunities and challenges with the use of RWE for regulatory decision making
- Explain 'objectivity' in the context of real-world (RW) studies and why it is important, and how emulating the scientific and operational processes from a gold-standard randomized, controlled clinical trial helps maintain objectivity in RW studies

Track: General Session

Session Chair(s)

Sarah Martin, PhD, MS

Senior Director - Global Regulatory Policy (Oncology)
Eli Lilly & Co., United States

Sarah currently serves as senior director of global regulatory policy for oncology at Eli Lilly. She previously served as senior director of science and regulatory advocacy for the Pharmaceutical Research and Manufacturers of America (PhRMA) where she led their regulatory advocacy efforts on key issues including real-world evidence. Sarah worked for the American Association for Cancer Research (AACR) where she led their efforts to modernize the regulatory process and engage in the development and implementation of programmatic and policy initiatives with the US FDA Oncology Center of Excellence.

Speaker(s)

Real-world Studies to Support Regulatory Decision-making: The What and the How?

Rohini Hernandez, PhD, MPH

Director, Center for Observational Research
Amgen, United States

Rohini Hernandez, PhD, MPH, is a Director of Observational Research in the Center for Observational Research at Amgen. She leads a Pharmacovigilance Epidemiology team focused on leveraging real world data to support benefit/risk assessments globally and across therapeutic areas. Prior to her current role, Rohini worked in the oncology therapeutic area at Amgen, where she designed and led observational studies in support of bone health among patients with solid tumors. Her research interests include exploring methods for controlling biases in pharmacoepidemiology studies, with a specific focus on identifying opportunities for improved assessment of product safety during pregnancy. She received her PhD in Epidemiology from Boston University.

Trial Emulation Beyond Study Design: Meeting Regulatory Standards for Objectivity in Real-World Studies

Brian Conroy, PhD, MS

Senior Director of Biostatistics
Aetion, United States

Brian Conroy is a biostatistician at Aetion, Inc. He received his PhD and MS in biostatistics from Emory University.

9:20 AM — 10:35 AM

Maryland Ballroom

Session 7: Unexpected Issues in Pharmacoepidemiology Studies Applying Natural Language Processing to Clinical Notes

Key to driving data fitness for regulatory decision-making is improving the usability of unstructured notes, which are rich with clinical nuance but difficult to curate and analyze. Using the Sentinel Innovation Center's Multi-source Observational Safety study for Advanced Information Classification using natural language processing (MOSAIC-NLP) project as a case study - this session will describe strategies to address unexpected methodological challenges and discuss how to advance the use of NLP of clinical notes to support population-based pharmacoepidemiology studies. This project is applying NLP to unstructured EHR data from over 100 million patients across more than 100 health systems to identify outcomes, extract confounders, and contextualize longitudinal data.

Learning Objective :

- Identify how to utilize clinical notes to create a representative NLP training sample
- Design the taxonomy for annotation of the training set
- Create transparency and reproducibility through appropriate documentation

- Assess model generalizability given health system differences
- Evaluate epidemiological issues when using NLP to analyze clinical notes

Track: General Session

Level: Intermediate

Session Chair(s)

Nirosha M. Lederer, PhD, MS

Head, US Government Partnerships; Senior Director, RWE Strategy
Aetion, United States

Nirosha Mahendraratnam Lederer, PhD is Head of US Government Partnerships at Aetion. In this role, she leads partnership opportunities with the US federal government and advises clients on RWE trends. Before joining Aetion, she led the RWE portfolio at the Duke Margolis Center for Health Policy including developing policies and strategies for increasing the usability and acceptance of RWD and RWE for regulatory and payment decision-making. She previously served as SME in Patient-Focused Drug Development at the US FDA Oncology Center of Excellence and worked at Avalere Health. Dr. Lederer served on Capitol Hill with the House Committee on Ways and Means Subcommittee on Health during the passage of the Affordable Care Act.

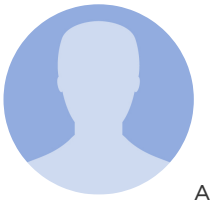
Speaker(s)

Speaker

Dena Jaffe, PhD

Lead Data Strategist
Oracle, Israel

Dr. Jaffe is a Real-World Data Strategist at Oracle. Her work involves the curation of the Oracle EHR RWD for real-world evidence. Dr. Jaffe has over 20 years of experience in epidemiology, quality of care, and RWE studies. Prior to joining Oracle, Dr. Jaffe was the deputy director of the Israel National Program for Quality Indicators in Community Healthcare and faculty at the Braun School of Public Health and Community Medicine, Hebrew University, Jerusalem. She has published across numerous therapeutic areas including cancer, migraine, depression, and Gaucher disease, with a focus on health inequalities. Dr. Jaffe received her PhD in epidemiology from Case Western Reserve University and a MS in pharmacology from Hebrew University.



Hasham Ul Haq

Machine Learning Engineer
John Snow Labs, United States

A Machine Learning Engineer with experience across various sectors, including healthcare. At John Snow Labs, my primary focus is to build scalable and pragmatic systems for NLP, that are both, production-ready, and give SOTA performance, which includes building bespoke models for different use-cases. Recently, a new direction of R&D is to adapt Large Language Models to different tasks with minimal error.

Speaker

Darren Toh, DrSc, FISPE

Professor
Harvard Medical School and Harvard Pilgrim Health Care Institute, United States

Darren Toh, ScD is DPM Endowed Professor in the Department of Population Medicine at Harvard Medical School and Harvard Pilgrim Health Care Institute. He is a pharmacoepidemiologist with an interest in the comparative safety and effectiveness research of medical products. His research has been focused on 1) assessing the risks and benefits of medical products using electronic data collected as part of routine healthcare delivery, and 2) developing and applying privacy-protecting analytic methods to conduct multi-center studies in distributed data networks. Darren is Principal Investigator of the FDA-funded Sentinel Operations Center.

10:35 AM — 11:15 AM Baltimore Ballroom

Refreshment and Networking Break

10:40 AM — 11:10 AM Homeland

Hosted Event/Non-CE: Case Study Spotlight hosted by Purpose Life Sciences: RWE Integration by Design: An Essential Component of Early RCT Design

Real-world data (RWD) and real-world evidence (RWE) often find their place in clinical development toward its conclusion. Results derived from them are often reserved for medical affairs, market access, or to bolster existing pre-planned clinical trials. However, this approach can create significant challenges, hindering the potential utility of RWE due to trial designs and protocols that may not align well with generating fit-for-purpose RWD. Today, there is growing momentum from regulators and scientists to shift towards a systematic integration of RWE during the initial trial design phase.

This presentation sheds light on the barriers that traditionally separate randomized clinical trials (RCTs) and RWE, highlighting the need for a more blended approach. Emerging concepts such as externally controlled trials and innovative designs like cohort multiple randomized controlled trials or trials within-cohorts are gaining traction as effective means to capture novel patient insights. By exploring the convergence of these diverse methodologies, we can uncover more meaningful and comprehensive insights than what each can achieve independently.

Learning Objective :

- Describe how current clinical trial design strategies and frameworks create barriers to RWE inclusion
- Explain the mechanics of blended trial and RWE approaches, and their potential to amplify the value of trial results
- Explore the operational and pragmatic implications for adopting integrated RWE and trial strategies

Track: Exhibitor Event

Session Chair(s)

EXHIBITOR

Sponsored Sessions

United States

Speaker(s)

Exhibitor

Dallas Hodgson

Vice President, Innovation Partnerships
Purpose Life Sciences, Canada

Dallas Hodgson is VP, Innovative Partnerships at Purpose Life Sciences. At PLS he is focused on providing life sciences partners and sponsors with clinical trial research services specializing in novel trial designs and augmented epidemiology. Dallas has over 30 years of experience working with real-world data (RWD) to generate evidence for Clinical, Regulatory, HEOR, Market Access, and Commercial functional groups.

11:15 AM — 12:30 PM Maryland Ballroom

Session 8: Cross-Industry Consortia Initiatives Addressing RWD Heterogeneity

While the number of studies utilizing RWD has increased significantly, there is a lack of standardized methodologies for capturing and analyzing real-world data (RWD) to generate real-world evidence (RWE). Coordinated efforts across the biopharmaceutical industry, academia, data vendors, and patient advocacy organizations can support robust use of RWD/E to generate lessons learned to promote consistency and standards in the field. This session will discuss cross-industry consortia and initiatives and early findings to advance the use of RWD/E.

Learning Objective : At the conclusion of this session, participants should be able to:

- Explain the need for collaborative studies focused on RWD/E methodologies and quality
- List examples of cross-industry RWE-related consortia, opportunities to engage, and lessons learned for future efforts
- Describe how results obtained from these activities promote a consistent approach for the robust use of RWD

Track: General Session

Session Chair(s)

Brittany McKelvey, PhD

Director, Regualtory Affairs
Friends of Cancer Research, United States

Speaker(s)

Early Field Experience From the AnCillary Studies to Evaluate EHR and Claims Real-World Data (ACE-RWD)

Meredith Nahm Zozus, PhD

Professor; Division Chief and Director, Clinical Research Informatics
University of Texas Health Science Center, United States

Meredith is a Professor, Division Chief and Scientific Director of Clinical Research Informatics for the University of Texas Health Science Center at San Antonio. She is the lead PI of the ACE-RWD program.

Harmonizing Guidelines on Post-Approval Observational Safety Studies for Medicines: Background, Status and Future of ICH M14

David Moeny, MPH, RPh

Acting Deputy Director, Office of Pharmacovigilance and Epidemiology, OSE/CDER
FDA, United States

David Moeny is a pharmacist and pharmacoepidemiologist with experience in clinical pharmacy practice, public health, drug utilization, regulatory pharmacoepidemiology, and international collaborations. At FDA, he has worked in both drug utilization and epidemiology teams and as the director for the Division of Epidemiology-II, participating in numerous evaluations of drug safety issues in Sentinel and other systems. He is currently serving as the Acting Deputy Director for the Office of Pharmacovigilance and Epidemiology in the Office of Surveillance and Epidemiology at the Food and Drug Administration, and is serving as the Rapporteur for the International Council of Harmonization M14 Guideline.

Assuring Audit and Inspection Readiness – Considerations for the Use of RWE/RWD in Regulatory Decision-making – Update from the TransCelerate Real World Data ARC Initiative

Abi Seifert, MBA

Global Head GCP and PV Country Quality
Novartis , United States

Abi has worked in the Pharma industry for over 25 years, serving in clinical development in both QA and operational roles. Her career started in clinical study monitoring and GCP auditing and evolved into roles that defined strategy around project management, process excellence and compliance in clinical development programs and in the use of RWE/RWD. She currently leads a team of QA associates in the oversight of GCP and PV quality in 60+ countries at Novartis. Abi holds a Masters in Management and is a Lean Six Sigma Blackbelt. She is currently a collaborator on the TransCelerate Audit Readiness Considerations team creating a set of recommendations for documentation of processes for programs using RWE/RWD in regulatory decision-making.

12:30 PM — 1:30 PM Baltimore Ballroom

Networking Luncheon

1:30 PM — 2:45 PM Maryland Ballroom

Session 9: Special Populations' Perspectives on RWD and RWE

Real-world data is of increasing interest and utility to medical product regulators seeking to observe and monitor treatment outcomes among patients in the real world. This is especially given that clinical trial settings are least reflective of typical settings in which clinically and socio-demographically diverse patients are monitored and treated. In addition, patients with rare diseases must often rely on real-world data to supplement, inform, or drive clinical trials with potential to substantiate timely and potentially life-saving therapies. This session will describe how real-world data is used by regulators to inform clinical trials and post-market strategies, generate insights around social determinants of health, and monitor outcomes in rare disease patients and populations underrepresented in clinical trials.

Learning Objective :

- Describe how real-world data is used to generate insights about social determinants of health and inform clinical trial and post-market strategies
- Discuss regulatory uses of real-world data to observe and monitor outcomes in populations that are underrepresented in clinical trials but commonly treated in the real world
- Demonstrate uses of real-world data sources reflective of and to serve rare disease populations

Track: General Session

Session Chair(s)

Rachele Hendricks-Sturrup, DrSc, MA, MSc

Research Director, Real-World Evidence
Duke-Robert J. Margolis, MD, Center for Health Policy, United States

Rachele Hendricks-Sturrup, DHSc, joins Duke-Margolis as the Research Director leading the Center's Real-World Evidence (RWE) portfolio, including managing its RWE Collaborative. She is a scientist/researcher, health policy and industry professional, journalist, and academician within the fields of health policy, business, and health innovation.

Speaker(s)

RWD and SDOH Generating Insights from Clinical Trials to Post Market Strategies

Jennifer Lamppa, PhD

Associate Vice President, Clinical Analytics
Inovalon, United States

Jen Lamppa serves as Inovalon's AVP of Clinical Analytics, leading solutions and services for real world data in clinical research. Jen has over a decade of experience in clinical research, analytics, and healthcare strategy, including: automated clinical study feasibility and operational reporting solutions, AI-enabled real-world data-based registries, health economics and outcomes research, and solution architecture for novel research models such as tokenized study data, hybrid studies, external control arms, and integrated data lake platforms. Jen earned her PhD in Biomedical Engineering from the Thayer School of Engineering and holds a Bachelor of Biomedical Engineering from Dartmouth College in Hanover, New Hampshire.

Optimizing Real-World Evidence in Rare Disease Registries

Victoria Hodgkinson, PhD

Chief Scientific Officer
Lumiio Inc., Canada

Dr. Hodgkinson is Chief Scientific Officer for Lumiio Inc. where she oversees the scientific management, operations, and coordination of national and global patient registries in rare diseases. Her expertise includes optimizing patient data collection programs through building and alignment of multi-stakeholder disease networks. She has participated in numerous studies provisioning rare disease RWE to regulatory and HTA bodies internationally. Additionally, Dr. Hodgkinson works with HTA bodies, such as CADTH, to support implementation of policies and best practices for use of registry data for regulatory decision-making and chairs a rare disease interest group with HTAi to improve value frameworks and RWE interpretation in rare diseases.

Regulatory Use of Real-World Data: Quantifying Drug Exposure Risk in Pregnancy with Retrospective Administrative Claims Data

Elizabeth Packnett, MPH

Lead Researcher, Real World Data Research & Analytics
Merative, United States

Elizabeth R. Packnett, MPH, is a lead researcher in the Real World Data Research and Analytics group at Merative supporting pharmaceutical and life sciences clients in the design and implementation of research studies. She has led several studies leveraging real world data for regulatory reporting including post-authorization safety studies to evaluate risk of prenatal exposures and evaluations of compliance with risk evaluation and mitigation strategies. The results of her research have appeared in numerous peer-reviewed publications related to maternal and child health, behavioral health, vaccination, and chronic respiratory disease. She holds an MPH in epidemiology from the University of Michigan School of Public Health.

2:55 PM — 4:10 PM Maryland Ballroom

Session 10: The Future of RWD and RWE

In the past two days, this conference has reviewed the proliferation of real world data sources and technological advances that have spurred innovative uses of real world data and real world evidence by sponsors in product development and regulators in product evaluations. In this session, we discuss the future trends for RWE. Potential topics may include:

- The use of RWE/RWD to address clinical post-marketing commitments issued by the US FDA for novel and supplemental NDA/BLA approvals in oncology
- FDA's framework for advancing RWE programs. What comes next?
- The concept of synthetic data, their applications, and challenges to adoption

Learning Objective : At the conclusion of this session, participants should be able to:

- Recognize trends in clinical post-marketing commitments (PMC) and requirements (PMR) issued by US FDA in oncology and how RWE can be applied
- Understand FDA's current framework for advancing RWE and identify areas for further development
- Describe the concept of synthetic data and assess opportunities for their application

Track: General Session

Session Chair(s)

Charles Lee, MBA, MS

Executive Regulatory Science Director
AstraZeneca, United States

Charles is currently Executive Regulatory Science Director at Astrazeneca. He oversees Global Regulatory science and strategy for therapeutic products in the renal, cardiovascular, diabetes, and NASH disease areas. Prior to this role, Charles was a Product Development Team Leader over a 10 year period at Astrazeneca and Roche where he led cross-functional project teams for programs in the early development phase (Ph0 to Ph2b). Previous to that, Charles spent 10 years in Global Regulatory affairs where he held leadership roles on programs across several therapeutic areas in different stages of development. Charles holds BS in Biology from The Johns Hopkins University, MS from University of Virginia, MBA from Columbia Business School

Speaker(s)

Regulatory Trends in Oncology Post-Marketing Commitments and Requirements issued by the US Food and Drug Administration

Adam Shiell, PharmD

Global Regulatory Strategist
Flatiron Health, United States

Adam has 10 years of regulatory affairs experience, focusing on oncology drug development. At Flatiron Health, Adam is the regulatory lead for prospective clinical research, working with sponsors on novel ways of using real-world evidence (RWE) to advance their portfolios. Prior to joining Flatiron, Adam worked in regulatory affairs at Eli Lilly & Company as a regulatory lead for oncology compounds in both early and late phase development.

Beyond the Cures Act: What's Next for FDA's RWE Program

Thomas David Brown, MD, MBA

Chief Medical Officer
Syapse, United States

Synthetic Data for RWD

Lucy Mosquera, MSc

Senior Director, Data Science
Replica Analytics, Canada

Lucy Mosquera has a background in biology and mathematics, having done her studies at Queen's University in Kingston and the University of British Columbia. In the past she has provided data management support to clinical trials and observational studies at Kingston General Hospital. She also worked on clinical trial data sharing methods based on homomorphic encryption and secret sharing protocols with various companies. At Replica Analytics, Lucy is responsible for integrating her subject area expertise in health data into innovative methods for synthetic data generation and the assessment of that data, as well as overseeing our analytics program.

4:10 PM — 4:25 PM

Maryland Ballroom

Closing Remarks

Closing Remarks

Session Chair(s)

Tamei Elliott, MS

Associate Director, Scientific Programs
DIA, United States

Tamei Elliott, MS, serves as the Associate Director of Scientific Programs for the Americas region at DIA. In this pivotal role, she is responsible for identifying and prioritizing content areas and topics crucial to DIA constituents. Tamei assesses the implications of significant regulatory and health policy changes, seamlessly integrating relevant content into the development and advancement of DIA conferences and courses. Her responsibilities extend to overseeing content development and strategy within the Americas region.

David Martin, MD, MPH

Vice President, Global Head RWE
Moderna, United States

David Martin leads Global RWE for Moderna. Previously he completed 20 years of active duty service split between the United States Air Force and Public Health Service. At the FDA he led the Division of Epidemiology in the Center for Biologics. Subsequently, he established the Real World Evidence group in the Office of Medical Policy to drive the agency's scientific, guidance, and submission review responses to the RWE provisions of the 21st Century Cures Act. He initiated key RWE pathfinding efforts with external stakeholders including RCT Duplicate and the open-source FDA MyStudies mobile application. He holds an MD and MPH from Johns Hopkins and is board certified in occupational & environmental medicine as well as clinical informatics.

4:25 PM — 4:25 PM

Conference Adjourns