Sheraton Philadelphia Downtown Hotel

Oct 24, 2024 7:30 AM - Oct 25, 2024 4:10 PM

201 North 17th Street, Philadelphia, PA 19103

Real-World Evidence Conference

Translating Insights into Real-World Value

Print Agenda

Day 1 Oct 24, 2024

7:30 AM — 5:30 PM — Liberty Ballroom Foyer (Ballroom Level)

Conference Registration

7:30 AM — 8:30 AM — Liberty Ballroom A

Networking Breakfast

8:30 AM — 8:45 AM — Liberty Ballroom B

Opening Remarks

Track: General Sessions

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, Head, PCO Center of Excellence
Novartis, United States

Dr. David Martin works across therapeutic areas as the Head of the Patient Centered Clinical Outcomes Center of Excellence at Novartis. Previously, he led Global RWE at Moderna, with accountability for provision of enterprise real world data and analytics as well as oversight for deliverables oriented toward regulators, payers, providers, and patients. He retired as a Captain after completing 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 RWE group to drive the agency's scientific, guidance, and submission review responses to the RWE provisions of the 21st Century Cures Act.

8:45 AM — 9:45 AM

Liberty Ballroom B

Session 1: A Year in Review

The session will explore new key milestones and major events in Real-World Evidence (RWE) since last year. The first presentation will provide a broad global overview of remarkable advances in RWE. The second presentation will summarize FDA's RWD/E guidances on drugs and biologics products and highlight the draft non-interventional study guidance, provide an update on RWE commitments for PDUFA VII, and offer insights on how FDA centers are leveraging RWE. The third presentation will discuss applications of RWE in FDA Sentinel, including the scope and progress of projects in the RWE Data Enterprise and Sentinel's PDUFA VII demonstration projects. The session will conclude with a panel discussion and audience Q&A.

Learning Objective :

- Discuss draft and final guidances on RWE from US FDA including draft guidance on non-interventional studies
- Describe FDA's Sentinel demonstration projects
- Recognize notable advances in RWE over the past year

Track: General Sessions

Session Chair(s)

SARAH K 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 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, clinical development, human drug review program, and CGTs. Sarah also 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)

Speaker

Gabriel I Innes, DVM, PhD

Health Science Policy Analyst, OMP, CDER
FDA, United States

Dr. Gabriel Innes is a Health Science Policy Analyst on the Real-World Evidence (RWE) Analytics Team in the Office of Medical Policy (OMP), Center for Drug Evaluation and Research (CDER), FDA. Responsibilities on the RWE Analytics Team include internal consults related to RWD and the generation of RWE, development of guidance documents related to RWE, as well as internal activities and policy related to the organization, analysis, and reporting of RWD/RWE. Dr. Innes joined OMP after completing the Centers for Disease Control and Prevention's Epidemic Intelligence Service Fellowship. He holds a PhD from the Johns Hopkins Bloomberg School of Public Health, and a VMD from the University of Pennsylvania School of Veterinary Medicine.

Sarah Dutcher, PhD, MS

Lead Epidemiologist, OSE, CDER
FDA, United States

Sarah Dutcher is the lead epidemiologist on the Sentinel Core Team in the Office of Surveillance and Epidemiology, Center for Drug Evaluation and Research, US Food and Drug Administration. In this role she manages the scientific components of the Sentinel System, CDER's active surveillance system for regulated medical products. Dr. Dutcher is a pharmacoepidemiologist and health services researcher with expertise in analysis of drug use and health outcomes in electronic healthcare data. Her background is multidisciplinary, involving the fields of epidemiology, pharmacy, health behavior, gerontology, economics, and health policy.

9:45 AM — 10:30 AM

Liberty Ballroom A

Refreshment and Networking Break

9:55 AM — 10:25 AM

Salon 5/6 (Mezzanine Level)

Hosted Session/Non-CE: Case Study hosted by OM1: Elevate Your Real-World Evidence Without Stretching Your Resources

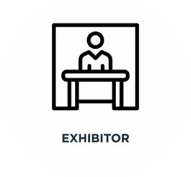
Discover how automated real-world data collection, driven by cutting-edge AI, is revolutionizing clinical research. Join OM1's Managing Director of Integrated Evidence Generation, Sonja Wustrack, as she delves into how these innovations are accelerating study timelines, cutting costs, and reshaping the future of prospective studies and registries. Learn how this advanced synergy of technology is sparking a paradigm shift in clinical research, paving the way for a more efficient and impactful era in life sciences.

Learning Objective : Featured Topics:

- LSS R&D
- Clinical Development
- HEOR
- RWD/RWE

Track: Exhibitor Event

Session Chair(s)



Sponsored Sessions

United States

Speaker(s)

Exhibitor

Eric Schrock

Chief Technology Officer
OM1, United States

Eric Schrock is the Chief Technology Officer at OM1, Inc. In this role, he is responsible for the Product Engineering, Data Engineering, IT, and Security teams that develop and operate OM1's technology platform and products. Prior to OM1, Eric was the CTO and Co-CEO at Jvion, a clinical AI company that uses prescriptive intelligence to improve patient outcomes. There, he ran the engineering, data science, professional services, and customer success teams

responsible for product development and delivery. Also, Eric was VP of Engineering and CTO at Delphix, a data virtualization company that simplifies the delivery of secure production data to enterprise teams.

10:30 AM — 11:45 AM Liberty Ballroom B

Session 2: Early Development Use Cases

This session will discuss the application of RWE in early clinical development and in supporting Regulatory decision-making. A case study will describe how external controls were used to augment the results of a single arm clinical trial in providing a perspective for program level decision making. Learnings from a review of FDA approvals during 2019-2023 on the uses of RWD in oncology applications will be presented. Examples and case studies of RWD use (other external control arms) in oncology regulatory applications will complement the first presentation on the use of external controls.

Learning Objective :

- Determine the appropriateness of external control data
- Describe how to create a statistically balanced external control
- Use an external control to help inform the results of a single-arm study
- To understand how real-world data (RWD) have successfully and unsuccessfully supported regulatory applications in oncology
- To identify learnings about ensuring impactful use of RWD in oncology clinical development and regulatory applications

Track: General Sessions

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)

Why and How: An External Control Used to Contextualize the Results of an Early Phase Single Arm

Metastatic Pancreatic Study

Lisa Ensign, PhD, MSc

VP, Statistics and Regulatory Science Innovation
Medidata, a Dassault Systemes Company, United States

Lisa Ensign is a VP of Statistics and Regulatory Science Innovation at Medidata. Her recent work is focused on creating robust analytical approaches to improve and transform the efficiency and rigor of clinical trials, centered on the use of external controls in areas of unmet medical need. Lisa began her career at MD Anderson Cancer Center and has extensive academic, industry. regulatory and entrepreneurial 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 teaches graduate courses on ethics and the responsible conduct of research.

Beyond External Control Arms: Utility of Real-World Data in Oncology Regulatory Applications

Ulka B Campbell, PhD

Head of Scientific Strategy
Aetion Inc, United States

Ulka Campbell is an epidemiologist and the Head of Scientific Strategy at Aetion, a healthcare technology and research services company, providing methods and regulatory support across therapeutic areas and leading research to inform regulatory RWE best practices. Previously, she was at Pfizer for 14 years leading regulatory studies and serving as the Head of Safety Surveillance Research, overseeing a team responsible for post-approval safety studies obligated to FDA and EMA. She has co-authored several publications and taught courses on pharmacoepidemiology, standards for decision-grade real world studies, causal inference, and epidemiologic methods, and is an Adjunct Assistant Professor of Epidemiology at Columbia University.

11:45 AM — 12:45 PM Liberty Ballroom A

Networking Luncheon

12:45 PM — 2:00 PM Liberty Ballroom B

Session 3: RWD Innovations in Late Phase and Postmarket Settings: A Review of Use Cases

Real world data can help answer a number of research questions across the drug development lifecycle using innovative study designs and technology. This session will explore recent use cases involving innovations in applying real world data in late phase and postmarket settings. A panel of experts from industry and RWD organizations will share learnings from

utilizing registry-based randomized controlled trials (R-RCTs) to improve drug development efficiency, implementing clinical trial linkage to RWD to gain deeper insights to a drug's benefits and risks, and exploring new ways for studying the medication safety during pregnancy through innovations in post-approval pregnancy safety studies (PA-PSS).

Learning Objective :

- Describe the current landscape using recent use cases of RWD innovations in the late phase and postmarket settings
- Describe the best practices for trial linkage and strategies to overcome some of the common challenges in implementation
- Incorporate regulatory considerations into the design of registry-based RCTs and PA-PSS

Track: General Sessions

Session Chair(s)

Camille Jackson

Director, Regulatory Policy; Legal and Regulatory
Flatiron Health , United States

Camille Jackson is the Director and Head of Regulatory Policy at Flatiron Health, bringing nearly 20 years of experience within policy and program management across various corners of the life sciences sector. Earlier in her career, Camille held roles at Clarivate, Sanofi, PhRMA, The World Bank, NIH, The George Washington University, and the American Academy of Child and Adolescent Psychiatry.

Speaker(s)

Implementing Clinical Trial Linkage to Real-World Data (RWD)

Mehdi Najafzadeh, PhD, MA, MS

Senior Director
Medidata Solutions, United States

Mehdi NajafZadeh, PhD, is Senior Director at Medidata AI. Before joining Medidata AI, he was an Assistant Professor of Medicine in the Division of Pharmacoepidemiology and Pharmacoeconomics, Department of Medicine, Brigham and Women's Hospital and Harvard Medical School, and the PI of large NIH and FDA funded projects that aimed at linking RCTs to RWD.

Utilize Registry-based RCT to Improve Drug Development Efficiency: DAPA-MI as a Case Study

Jingyu (Julia) Luan, PhD

Executive Regulatory Science Director, BioPharmaceuticals R&D
AstraZeneca, United States

Dr. Jingyu (Julia) Luan is an Executive Regulatory Science Director in AstraZeneca, overseeing the global regulatory strategy and supporting the research, development and commercialization of CVRM products. She is a core member of CVRM Regulatory Leadership Team. Prior to AZ, she worked at FDA for 13 years and held positions with increasing responsibilities, including Statistical Reviewer, Team Leader, and Acting Deputy Division Director. She had extensive experience in multiple therapeutic areas for both new and generic drugs. In addition, she was a research faculty member at Johns Hopkins University and a statistical consultant at the University of Kentucky. Dr. Luan is President of Chinese Biopharmaceutical Association 2023-2024.

Post-Approval Pregnancy Safety Study (PA-PSS) Innovations: New Ways for Studying the Safety of Medications During Pregnancy

Krista Marie Schroeder, MHS, PhD

Director, Global Patient Safety, Pharmacoepidemiology
Eli Lilly and Company, United States

Dr. Krista Schroeder is a pharmacoepidemiologist with 20 years of diverse research experience in the pharmaceutical industry spanning all phases of drug development from pre-clinical to post-marketing. Her expertise includes designing and executing real world evidence (RWE) observational studies for both reimbursement (health outcomes) and regulatory (pharmacoepidemiology) purposes. She is a recognized subject matter expert in the design of studies using secondary data and has multiple publications in this space. She is the principal scientist responsible for several post-authorization safety studies executed as commitments to regulatory bodies globally.

2:10 PM — 3:25 PM Liberty Ballroom B

Session 4: Real-World Data Standards for Regulatory Submissions: Exploring the Challenges, Solutions, and Potential Alternatives

In this session panelists will share new developments in real-world data (RWD) standards for regulatory submissions, including: updates from sponsors, data providers, standards development groups, validation organizations, and the FDA. Topics will include challenges, solutions, and potential alternatives to data standards from FDA's Catalog and assess other common data models that could be considered as additions.

Learning Objective :

- Explain the importance of real-world data standards for regulatory submissions and generally why existing standards used for clinical trials is not currently a perfect fit
- List potential RWD alternatives to existing regulatory data standards along with pros and cons of each
- Describe challenges faced when developing and utilizing standards for Real-World Data

Track: General Sessions

Level: Intermediate

Session Chair(s)

Diane Gubernot, DrPH, MPH

Epidemiologist
US FDA, United States

Dr. Diane Gubernot is a Health Scientist in the Office of Biostatistics and Pharmacovigilance at the Center for Biologics Evaluation and Research (CBER) of the USFDA. She conducts regulatory reviews of RWE/RWD submitted in biologic product applications and performs research to help support RWE policy development as well as other public health initiatives. Dr. Gubernot has been with the FDA for more than 30 years and has worked as an Investigator in the Office of Regulatory Affairs, a Team Lead Epidemiologist in the Office of the Commissioner, and a regulatory product reviewer at CBER.

Speaker(s)

RWE Standards: Reliability and Relevance

Dan Riskin, MD, MBA

Founder and Chief Executive Officer
Verantos, United States

Dan Riskin is the founder and CEO of Verantos, the global leader in high-validity real-world evidence at scale. Recognized across the globe as an expert in healthcare AI, Dr. Riskin has developed products that influence the care of millions of patients annually. His advocacy includes testimony before Congress on the 21st Century Cures initiative and serving on the Health Advisory Committee for two presidents. He is Clinical Professor of Surgery at Stanford University.

Submission Standards for RWD: Gaps, Limitations, and Recommendations

Sarah Ferko, MS, PMP

Senior Managing Consultant, Artificial Intelligence & Analytics, U.S. Federal
IBM, United States

Sarah Ferko is a Senior Managing Consultant and Project Management Professional (PMP) in IBM's Artificial Intelligence and Analytics service line within the Data and Technology Transformation practice area supporting US Federal projects within IBM Consulting. Sarah has supported multiple offices at the FDA since 2017, including CDER OB, OSP, OCS, and OND. Sarah holds a B.S./M.S. in Applied Mathematics from the University of Akron in Akron, Ohio.

Interpreting and Implementing RWD Standards – Challenges, Opportunities, and Open Questions

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 15 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.

Panelist

G. Scott Gordon, PhD

Senior Health Informatics Officer, OSP, CDER
FDA, United States

Since 2016, Dr. Gordon has been a lead for data standardization efforts including those for pharmaceutical quality, manufacturing, and labelling, as well as real-world data derived from health information technology for use in clinical research and pharmacovigilance. Before arriving at FDA, Dr. Gordon received his core scientific training with a Ph.D. in Molecular Microbiology from Tufts University Medical School, entered the public health domain in 2005 working on public health emergency preparedness and from 2011 with a focus on public health informatics.

3:25 PM — 4:00 PM Liberty Ballroom A

Refreshment and Networking Break

3:30 PM — 4:00 PM Salon 5/6 (Mezzanine Level)

Hosted Session/Non-CE: Case Study Sponsored by Verantos: Asthma and IBD on the Impact of Curation and Enrichment for Rich and Reliable RWD

This case study will illustrate how curation and enrichment are necessary means of acquiring rich and reliable data. An example from our work in asthma will demonstrate the importance of measuring accuracy, as well as the impact of curation and enrichment, on both identifying a relevant population and measuring its outcomes. An example from our work in inflammatory bowel disease will demonstrate the use of enrichment to infer outcomes that rely on survey measures that are not routinely collected.

Learning Objective : Featured Topics:

- How curation and enrichment affect both the study population and the measured outcomes of it
- How enrichment can fill gaps in explicit outcomes that may be commonly captured in trials but are uncommon in the real world
- How measuring accuracy, completeness, and traceability matter beyond regulatory audiences

Track: Exhibitor Event

Session Chair(s)

EXHIBITOR

Sponsored Sessions

United States

Speaker(s)

Exhibitor

Tim Wolfe

Vice President of Product Management
Verantos, United States

4:00 PM — 5:15 PM

Liberty Ballroom B

Session 5: Health Equity in Drug Development: Leveraging RWD to Inform and Improve Diversity

Historically, clinical trials lack inclusion and representativeness that may hinder the generalizability of results to the intended patient population and contribute to existing health inequities. Real-world data (RWD) can provide context and insights both to set appropriate goals for diversity in clinical research, as well as provide additional information on diverse patient populations. This session will discuss the types of data and evaluation of appropriate data sources to define diversity, inclusion, and representativeness, with a special focus on Social Determinants of Health and health equity, and how to incorporate RWD to enhance drug development for a more comprehensive view of the population of interest.

Learning Objective :

- Identify what factors should be captured in RWD sources to define representativeness, diversity, and inclusion
- Describe how to leverage RWD and mitigate biases to generate insights on diversity and representativeness for a variety of research objectives in drug development
- Demonstrate example applications of RWD to build diverse and inclusive study populations across drug development strategies

Track: General Sessions

Session Chair(s)

Brittany Avin McKelvey, PhD

Director, Regualtory Affairs
Friends of Cancer Research, United States

Brittany Avin McKelvey serves as the Director of Regulatory Affairs at Friends of Cancer Research (Friends). Friends is an advocacy organization based in Washington, DC that drives collaboration among partners from every healthcare sector to power advances in science, policy, and regulation that speed life-saving treatments to patients. Brittany supports the development and implementation of the organization's research and policy agenda. She leverages her patient advocacy and scientific background to generate scientific evidence and policies to advance progress in oncology care. She collaborates with diverse stakeholders to inform policy objectives and execute evidence-based research projects.

Speaker(s)

Representative of What? Pragmatic Strategies to Improve Diversity and Inclusion When the Research Questions Demand it

Melinda Baker, PhD

Director of Dataset Strategy & Commercialization
Mission Health Labs, Inc. (dba PicnicHealth), United States

Melinda Baker, PhD is the Director of Dataset Strategy and Commercialization at PicnicHealth. Melinda is passionate about empowering patients to take ownership of their medical records and helping life science partners infuse patient-centric methods into their research practices. She has dedicated her career to building new technologies and products that help researchers accelerate medical discoveries. Before moving to industry, Melinda obtained a doctorate from Princeton University and completed post-doctoral research at the University of Medicine and Dentistry of New Jersey. Her academic interests focused on using systems and structural biology techniques to understand signaling pathways of cellular motility and gene transfer.

Discovering Patient Barriers in Real-World Data to Inform Diversity Strategies

P. Karina D'Angelo, PhD

Director, Scientific Data Strategy
Parexel, United States

Lessons Learned: Health Equity in Medicare Claims Data Research

Yun Lu, PhD, MS

Deputy Division Director, DABRA, OBPV, CBER
FDA, United States

Dr. Yun Lu is the Deputy Division Director for the Food and Drug Administration (FDA)/Center for Biologics Evaluation and Research (CBER)/Office of Biostatistics and Pharmacovigilance (OBPV)/Division of Analytics and Benefit-Risk Assessment (DABRA). 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).

5:15 PM — 6:15 PM | Liberty Ballroom A

Networking Reception

Day 2 Oct 25, 2024

7:30 AM — 4:10 PM | Liberty Ballroom Foyer (Ballroom Level)

Conference Registration

7:30 AM — 8:00 AM | Liberty Ballroom A

Networking Breakfast

8:00 AM — 9:15 AM | Liberty Ballroom B

Opening Remarks and Session 6: Sand in Your Shoes? The Nitty-Gritty in Generating Regulatory-Grade RWE Using

Emerging Data Sources and New Platforms: Global Perspectives

The number of real-world data (RWD) sources available to generate real world evidence (RWE) has been increasing rapidly in recent years. The increasing availability and ease of access to data has been accompanied by numerous guidance documents and position papers among regulators, health technology assessment (HTA) bodies, and others that provide principled approaches to assessing fit-for-purpose RWD. While these documents and papers have enabled innovation in practice, they are accompanied by complexities that warrant the need for both pragmatism and close partnership and alignment of expectations among RWE developers and end-users.

Learning Objective :

- Describe early signs of alignment on RWE standards, considerations, and definitions among regulators globally, including ongoing developments
- Describe the need for current guidance documents to be applied pragmatically to balance data innovation with scientific rigor
- escribe environmental, system, and study-specific challenges to leveraging RWE and solutions that balance RWD limitations with opportunities to improve population and patient health

Track: General Sessions

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

Dr. Rachele Hendricks-Sturrup is the Research Director of Real-World Evidence (RWE) at the Duke-Margolis Institute for Health Policy in Washington, DC, strategically leading and managing the Institute's RWE Collaborative and RWE policy research portfolio and education. As an engagement expert, biomedical researcher, bioethicist, and policy practitioner with over 18 years of experience, her work centers on addressing implementation, regulatory, and ethical, legal, and social implications (ELSI) at the intersection of health policy and innovation. She presently partners with Duke University faculty, scholars, students, and external practicing experts to advance the Institute's biomedical innovation work.

Speaker(s)

Speaker

Lina Titievsky, PhD, MPH

Senior Director and Head of Hepatology Epidemiology
GlaxoSmithKline, United States

Lina Titievsky, MPH PhD, is a head of Hepatology and Classic and Established Products Epidemiology in Global Epidemiology at GSK. Prior to joining GSK, Lina has worked across multiple therapeutic areas including drugs, vaccines, and cell & gene therapies. Prior to joining GSK, Lina's professional journey included her work as an epidemiologist at Pfizer, Intercept and Vertex. In her current role, she leads a group of epidemiologists responsible

for generating evidence ranging from support of discovery and pre-clinical through regulatory approval. Lina holds a PhD in Epidemiology from Columbia University.

Speaker

Christian Hampp, PhD, FISPE

Senior Director, Pharmacoepidemiology
Regeneron, United States

Dr. Hampp is a Senior Director, Pharmacoepidemiology at Regeneron Pharmaceuticals, Inc., where he leads the oncology and hematology therapeutic areas. Before joining Regeneron in 2021, he worked for over 10 years as a senior epidemiologist in the Office of Surveillance and Epidemiology in FDA's Center for Drug Evaluation and Research. Dr. Hampp is also an Affiliate Clinical Associate Professor at the University of Florida, College of Pharmacy. He received his pharmacy degree from Saarland University in Germany, and a PhD in pharmacoepidemiology from the University of Florida.

Speaker

Jeffrey Brown, PhD

Chief Scientific Officer
TriNetX, United States

Jeffrey Brown, PhD, Chief Scientific Officer at TriNetX and Lecturer (parttime) at Harvard Medical School (HMS), is an internationally recognized expert in the use of real-world data to support the evidentiary needs of regulatory agencies and medical product sponsors. He has 25+ years of research experience using real-world data, most recently as an Associate Professor in the Department of Population Medicine (HMS) and a trusted consultant to numerous research groups and pharmaceutical companies. At HMS he served as the Lead Data Scientist for the FDA Sentinel Operations Center and as PI for several multi-site pharmacoepidemiologic studies to support FDA and EMA regulatory requirements.

Speaker

Mina Tadrous, PharmD, PhD, MS

Assistant Professor
University of Toronto, Canada

Mina is an assistant professor at the Leslie Dan Faculty of Pharmacy at the University of Toronto and the Tier 2 Canada Research Chair in Pharmaceutical Policy and Real-world Evidence. He is also co-director of Pharmaceutical Policy and Pharmacy Practice at the Ontario Drug Policy Research Network (ODPRN) and ICES adjunct scientist. Mina leads research focused on evaluating drug policies and post-marketing surveillance of medications. He works closely with policymakers and uses large data sets to answer questions about real-world safety and effectiveness and improving the optimal use of medications.

9:20 AM — 10:35 AM Liberty Ballroom B

Session 7: Methodological Insights on Aspects of Non-Interventional Studies

This session will highlight specific methodological issues relevant to using real-world data to generate real-world evidence. The first presentation will discuss the history and evolving landscape of causal methods including propensity scores, g-computation, and methods based on machine learning. The second presentation will discuss the test-negative design derived from the case-control study design. The third presentation will discuss negative control outcome studies designed to assess comparability of potential treatments groups. 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:

- Summarize basic concepts, applications, and methods of causal methods
- Describe advantages and pitfalls of studies using negative control outcomes
- Identify major methodological challenges when planning or evaluating a test-negative study design

Track: General Sessions

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)

Strengths and Challenges of Test-Negative Designs to Assess Post-Marketing Vaccine Effectiveness

Delphine Saragoussi, MD, MSc

Executive Director, Epidemiology and Scientific Affairs
PPD, part of Thermo Fisher Scientific, France

Executive Director in the RWE group at Evidera, PPD, part of Thermo Fisher Scientific, in Paris, France. Dr. Saragoussi is a physician specialized in Public Health and epidemiologist. In the pharmaceutical industry during 12 years and then as a consultant for the past 6+ years, she has developed and implemented various RWE plans to support market access. Her research methods expertise covers the collection of primary data as well as the use of electronic

databases. It includes natural history studies, burden of illness evaluations, treatment patterns descriptions, PRO validations, real-world effectiveness studies. Dr. Saragoussi is also well versed in the generation of RWE to meet regulatory needs in terms of safety and effectiveness.

Using Negative Outcome Control Studies to Assess Study Validity in the Real-World

David Pritchard, PhD

Director, Data Management & Statistics
Target RWE, United States

David Pritchard is a Director, Data Management & Statistics at Target RWE, where he is responsible for overseeing the statistical methodology utilized for and implemented in Target RWE's collaborative research projects. David's research interest include causal inference, negative control outcomes, machine learning with health care outcomes, and statistical software systems.

Traditions and Frontiers in Causal Methods - From the Rise and Fall of Propensity Scores to Causal AI for RWE

Andrew Wilson, PhD, MS

Head of Innovative RWD Analytics
Parexel, United States

As the Head of Innovative RWD Analytics at Parexel, I have the privilege of collaborating broadly on the frontiers of using real-world data, emphasizing the importance of context within the 'data generation process.' My recent collaborations intersect machine learning, causal inference, and reproducibility in research. Working within the evolving regulatory landscape, which is increasingly open to modern, established methods, I aim to help advance the field of real-world data analytics. I hold an adjunct appointment at the University of Utah and maintain affiliations with various institutions to maximize collaborative opportunities.

10:35 AM — 11:15 AM Liberty Ballroom A

Refreshment and Networking Break

10:40 AM — 11:10 AM Salon 5/6 (Mezzanine Level)

Hosted Session/Non-CE: Case Study hosted by Parexel: Transforming Evidence Generation: Scalable Solutions for Complex Requirements

Parexel is conducting a post-authorization safety study (PASS) and registry build to assess the long-term safety of a drug treating a rare neurological disease. The study, mandated by EMA's PRAC, aims to characterize and quantify potential risks and address queries from European regulators. With the development of a novel data ingestion and analysis engine to process over 15 disparate sources, data is transformed into a common model for segmentation and analysis. This creative approach allows our customer to effectively assess product safety in line with regulatory mandates while maintaining access to the novel medicine for the rare and vulnerable patient population.

Learning Objective : Featured Topics:

- Innovative frameworks and infrastructure for all stakeholder needs
- Integrated evidence platforming across the product lifecycle
- Best practices for scalable evidence generation

Track: Exhibitor Event

Session Chair(s)

EXHIBITOR

Sponsored Sessions

United States

Speaker(s)

Exhibitor

Mike D'Ambrosio

Senior Vice President and Global Head, Real World Research (RWD/RWE)
Parexel, United States

11:15 AM — 12:30 PM

Liberty Ballroom B

Session 8: Real-World Data in the Real World: How to Operationalize the Data Journey

The expectations from regulators and other decision-making stakeholders are increasing for the use of real-world data to generate valid and robust evidence. Putting theory into practice has evolved using new technology and methods to improve our operational execution of real-world study protocols. This session will provide practical applications for the data journey from abstraction, linkage, programming and reporting to increase the quality of real- world evidence generation.

Learning Objective :

- Understand the benefits and risks of human-only, AI-only, and human-in-the-loop AI-enabled data abstraction
- Apply concepts from software engineering to avoid errors throughout the lifecycle of a study and ensure reproducibility
- Describe considerations and demonstrate data management steps for linking administrative claims data with clinical trial data using tokenization

Track: General Sessions

Session Chair(s)

Jaclyn Bosco, PhD, MPH, FISPE

Vice President, Global Head of Epidemiology & Database Studies
IQVIA, United States

Dr. Jaclyn Bosco Global Head of Epidemiology in Real World Solutions at IQVIA, is responsible for driving real-world evidence (RWE) generation for regulators, clinicians, patients and payers using passive and primary data collection through clinicians and person-generated health to support the safety and effectiveness of drugs, biologics, and medical devices from early clinical development through the post-approval phase. She identifies the best approach for capturing data on a global scale as well applies local approaches to address market-specific needs. As a thought leader in real world research, she is invited to speak at international congresses and sits on scientific advisory boards and committees.

Speaker(s)

AI-Enabled Data Abstraction: Benefits and Risks for Data Curation

Troy Astorino

CTO
PicnicHealth, United States

Troy Astorino, co-founder and Chief Technology Officer of PicnicHealth, holds degrees in Aerospace Engineering and Physics from MIT. At PicnicHealth, Troy spearheads the development of technologies transforming unstructured medical records into research datasets. His team has pioneered AI for data standardization, enabling large-scale observational studies. Troy's work focuses on enhancing data quality and interoperability in real-world evidence generation. He has implemented systems for continuous data auditing, significantly improving research reliability. PicnicHealth collaborates with pharmaceutical companies and research institutions to accelerate drug development and improve patient outcomes.

Seeing Eye to Eye: Enhancing Inter-Team Communication for Reproducible Real World Evidence

Nuvan Rathnayaka

Manager of Biostatistics
Target RWE, United States

Supplementing the Identification of Clinical Endpoints through Tokenization of Clinical Trial Participants

Claudia Salinas, PhD

Senior Director, GPS Pharmacoepidemiology
Eli Lilly and Company, United States

Dr. Salinas is a pharmacoepidemiologist, with a doctorate in Epidemiology from the University of Washington, currently working in late stage and postmarketing clinical development at Eli Lilly and Company. She has expertise in real-world data analysis and causal inference focusing on drug development and the comparative safety of medications, particularly for auto-immune disorders. In previous work she was engaged in prostate cancer research, including evaluation of genetic risk factors from inflammatory pathways. Her current interests include linkage of RWD and clinical trial data to enhance the evaluation of drug safety. She is an advocate for communication about pharmacoepidemiology careers in industry and welcomes trainee questions.

12:30 PM — 1:30 PM Liberty Ballroom A

Networking Luncheon

1:30 PM — 3:00 PM Liberty Ballroom B

Session 9: Is the Future Here, Near, or Neither? Exploring the Intersection of AI and RWD in Pharmacoepidemiology

This session will highlight current innovative explorations when applying artificial intelligence to RWD sources. The first presentation will share lessons learned and opportunities for extracting and incorporating data from unstructured EHR clinical notes using NLP into structured data to improve the validity of pharmacoepidemiology studies. The second presentation will discuss principles and present case studies around using generative AI to transform the design of clinical studies. The third presentation delves into the details of using ML to optimize patient selection for clinical trials as viewed from a personalized medicine lens. We will wrap up with an exploration of numerous policy principles that can inform the development of effective policy and regulatory frameworks on the intersection of RWD and AI.

Learning Objective :

- Demonstrate the benefits and challenges of using information extracted from clinical notes using NLP in a pharmacoepidemiology study
- Better understand the role of generative AI in optimizing clinical study designs
- Evaluate the potential of ML to enable predictive treatment effect modeling from real-world data
- Contemplate the implications of generative AI from a policy perspective

Track: General Sessions

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.

Speaker(s)

Natural Language Processing in Pharmacoepidemiology: Lessons from the Multi-source Observational Safety study for Advanced Information Classification using NLP (MOSAIC-NLP)

Jenna Wong, PhD, MSc

Assistant Professor, Department of Population Medicine
Harvard Medical School and Harvard Pilgrim Health Care Institute, United States

Jenna Wong, PhD, is an Assistant Professor in the Department of Population Medicine at Harvard Medical School and Harvard Pilgrim Health Care Institute. She received her PhD in Epidemiology from McGill University and previously worked at the Institute for Clinical Evaluative Sciences in Ontario, Canada. She has nearly 15 years of experience conducting epidemiological studies using real-world data from linked electronic health databases. Her research uses machine learning techniques for different predictive modeling applications in epidemiology, where she is currently using machine learning and artificial intelligence to extract information from unstructured text to enhance the ability to use real-world data to study off-label drug use.

Unleashing RWD and RWE: Transforming Clinical Study Design with Generative AI

Susant Mallick, MBA

Founder and CEO, Life Sciences Practice Leader
Cloudhub BV, Netherlands

Susant Mallick comes up with 23+yrs of Pharma and IT background on building disruptive solutions/products in Clinical and Regulatory space. He is technology evangelist on cutting edge technology like (Artificial Intelligence, Machine Learning, IoT, Cloud etc) and an industry leading speakers across geographies. He has been working with various customers and partners in pharma and healthcare to drive digital transformation in clinical and regulatory landscape. He was instrumental in implementing/building many Regulatory solutions using advanced technology. Innovation and Digital Transformation in Healthcare and Life Sciences are two key focus areas.

Leveraging RWD to Enable Predictive Treatment Effect Modelling in Personalized Medicine

Flavio Dormont, PhD, MBA, MS

Therapeutic Area Head, Clinical Development RWE
Sanofi, United States

The Role of Real-World Data in AI Policy

Emilie Scherrer, MS

Head of Health Economics & Outcomes Research
Tempus AI, United States

Emilie Scherrer is the Head of Health Economics & Outcomes Research at Tempus AI and is a member of the RWE Alliance. Emilie was previously the Global Director of Health Economics and Outcomes Research at Seagen. She has worked in biopharma (Amgen, Merck, Seagen, Pfizer) for a decade in roles across HEOR and RWE positions. She has extensive knowledge in designing and executing non-interventional, observational research studies for external dissemination (40+ publications) and regulatory submissions. Emilie earned an undergraduate degree from USC and a Masters in Health Economics from the London School of Economics / LSHTM.

3:00 PM — 4:00 PM

Liberty Ballroom B

Looking Forward and Closing Remarks

Track: General Sessions

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, Head, PCO Center of Excellence
Novartis, United States

Dr. David Martin works across therapeutic areas as the Head of the Patient Centered Clinical Outcomes Center of Excellence at Novartis. Previously, he led Global RWE at Moderna, with accountability for provision of enterprise real world data and analytics as well as oversight for deliverables oriented toward regulators, payers, providers, and patients. He retired as a Captain after completing 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 RWE group to drive the agency's scientific, guidance, and submission review responses to the RWE provisions of the 21st Century Cures Act.

Speaker(s)

4:00 PM — 4:00 PM

Conference Adjourns