

# Estimands in Real-World Evidence Studies

## Abstract

This webinar explored the pivotal role of estimands in real-world evidence (RWE) studies, bridging the gap between regulatory guidance and practical implementation. The session addressed challenges unique to RWE settings, such as heterogeneous patient populations, complex treatment regimens, and the impact of intercurrent events on study outcomes with a focus on generating RWE that can inform regulatory decision-making. Through practical examples, case studies, and an engaging panel discussion featuring domain experts, the session highlighted best practices for defining estimands that enhance the interpretability and reliability of RWE findings. This session aimed for participants to leave with a systematic approach to estimand definition, empowered to conduct RWE studies that are robust, actionable, and aligned with evolving regulatory standards.

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## Speakers

### **Ibrahim Turkoz, *Johnson & Johnson* (Moderator)**

Ibrahim Turkoz, PhD, is a Scientific Director at Johnson & Johnson Innovative Medicine R&D. With over 25 years of clinical research experience in the pharmaceutical industry, he has worked across all phases of clinical drug development. Prior to joining J&J, Dr Turkoz held positions at several leading pharmaceutical consulting firms.

His current research interests encompass causal inference and comparative effectiveness, and he employs innovative methodologies for the design and execution of pragmatic trials and prospective observational studies. Dr Turkoz is a founding member of The International Society for CNS Clinical Trials and Methodology. He has authored and co-authored over 80 articles in peer-reviewed journals.

### **Hongwei Wang, *AbbVie***

Dr Hongwei Wang is a Sr. Research Fellow and Immunology TA Head, Medical Affairs & Health Technology Assessment Statistics at AbbVie. Prior to that, he worked at Sanofi and Merck. Hongwei received his PhD in Statistics from Rutgers University and his research interests include real-world studies, network meta-analysis, advanced analytics and their application to different stages of drug development.

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## Speakers - Continued

### **Hana Lee, FDA**

Hana Lee, PhD, is a Senior Statistical Reviewer in the Office of Biostatistics (OB) at the Center for Drug Evaluation and Research (CDER), FDA. She leads and oversees FDA-funded projects that support the development of the agency's real-world evidence (RWE) programme.

She also serves as a co-lead of the RWE Scientific Working Group of the American Statistical Association (ASA) Biopharmaceutical Section, an FDA public-private partnership involving scientists from the FDA, academia and industry to advance the understanding of real-world data (RWD) and RWE to support regulatory decision-making. In 2024, Hana received the FDA's most prestigious award for excellence in advancing and promoting statistical innovation in the use of RWD/RWE for regulatory decision-making.

### **Birol Emir, Pfizer**

Birol Emir, PhD, is Executive Director and Head of Real-World Evidence (RWE) Statistics at Pfizer. He is a Fellow of the American Statistical Association and has served as Adjunct Professor of Statistics and as a lecturer at Columbia University in New York. His primary focuses have been on real-world evidence generation, predictive modelling, and data analysis.

Birol has numerous publications in refereed journals and co-authored *Interface between Regulation and Statistics in Drug Development* (Alemayehu, Emir and Gaffney 2021, CRC Press). He has also co-edited a book to fill the gap in health economics and outcomes research. Birol has given many invited talks and short courses at statistical and clinical conferences.

### **Ben Ackerman, Johnson & Johnson**

Ben Ackerman is a Principal Biostatistician at Johnson & Johnson Innovative Medicine, where he provides support across therapeutic areas to design and analyse randomised trials, namely those that combine trial data with real world data. He has expertise in causal inference methods to address biases in both randomised trials and non-experimental studies. He is also a lead researcher on a recently awarded FDA U01 grant to develop novel statistical methods for mitigating endpoint measurement error bias between trial and real world settings when conducting externally controlled trials. Previously, Ben worked as a Quantitative Scientist at Flatiron Health, an oncology real world data vendor, where he oversaw the design of studies leveraging EHR data to improve cancer care in the United States. Ben holds a PhD in Biostatistics from the Johns Hopkins Bloomberg School of Public Health.