Biostatistical Considerations When Using RWD and RWE in Clinical Studies for Regulatory Purposes

Abstract

Real-world evidence (RWE) has emerged as a valuable source of information in clinical practice and drug development, offering the potential to optimise access to innovative medical products, especially for rare populations in need. As a result, regulatory agencies, public–private partnerships and health technology assessment organisations have launched major initiatives and issued guidance on the use of RWE in regulatory decision-making.

This webinar aims to examine the key biostatistical challenges and methodologies associated with leveraging RWE for clinical trials and medical product development, drawing on two companion articles. The first article provides an overview of the current landscape of using RWE to inform clinical study design and analysis, while the second article focuses on the practical challenges in the design and analysis of studies employing RWE.

The webinar will discuss the potential applications of RWE in regulatory decision-making, driven by a statistical perspective. It will highlight the role of the estimand framework in studies using RWE and how integrating the target trial emulation (TTE) framework supports the implementation of estimand thinking in the study design and analysis process. It will also look at practical considerations relevant to studies using RWE, for identifying methodological gaps that need to be addressed in future work, and emphasise the necessity for evaluating applied examples to guide practitioners in this evolving field.

The webinar will be of interest to statisticians, researchers, regulatory professionals and stakeholders involved in medical product development. It will offer insights into the use of RWE and the statistical considerations for more robust evidence generation and decision-making.

Speakers

Chantal Quinten, *European Medicines Agency*

Chantal Quinten is a senior expert in health data analytics and real-world data (RWD) at the European Medicines Agency, contributing to the Real-World Evidence (RWE) workstream of the Data Analytics Task Force (TDA-RWE). Chantal leads the TARGET-EU initiative at the European Medicines Agency, driving the integration of target trial emulation and the estimand framework to strengthen causal inference and the regulatory use of real-world evidence (RWE). She drives key initiatives to enhance the use of RWD, supporting methodological development and evidence-based regulatory approaches. Chantal has held pivotal roles at the European Centre for Disease Prevention and Control and the European Organisation for Research and Treatment of Cancer, enhancing disease surveillance systems and advancing the use of clinical and patient-reported data.

Evgeny Degtyarev, *Novartis*

Evgeny Degtyarev is Global Program Biostatistics Head at Novartis, where he leads a team of quantitative scientists supporting CAR T programmes in haematology. He joined Novartis in Basel in 2013 and has since contributed to multiple oncology programmes across targeted and immunotherapies. Evgeny co founded and co led the industry working group Estimands in Oncology (2018–2024; www.oncoestimand.org), which operated as an EFSPI/PSI Special Interest Group and an ASA Biopharmaceutical Section Scientific Working Group, including a dedicated RWE task force focused on applying estimands and target trial frameworks to real world data.

Yixin Fang, *AbbVie*

Yixin Fang is a Director of Medical Affairs and Health Technology Assessment Statistics (MA&HTA Statistics) and a senior research fellow at AbbVie. His research interests include causal inference and machine learning with applications in clinical trials and real-world studies. He published a book titled Causal Inference in Pharmaceutical Statistics in 2024. In addition, Yixin is an ASA fellow and an associate editor for Statistics in Biopharmaceutical Research.

Biostatistical Considerations When Using RWD and RWE in Clinical Studies for Regulatory Purposes

Moderators/Organisers

Beilei He, *Bristol Myers Squibb*

Beilei He, BSc, MSc, is an Associate Director of Biostatistics at Bristol Myers Squibb, with extensive experience in the design and analysis of clinical trials and real-world evidence (RWE) studies. His expertise spans oncology, immunology and rare diseases, with a strong focus on leveraging real-world data (RWD) to support innovative study design, as well as regulatory and market access strategies. His research interests include methodological innovation, causal inference, estimands in RWE, and the integration of RWD into clinical development. Beilei is an active contributor to cross-industry initiatives and regulatory engagements focused on the use of RWD for decision-making.

Leanne Goldstein, *Amgen*

Leanne Goldstein is a Biostatistics Manager in oncology, with experience in Phase I–III clinical trials. She has over 20 years of biostatistics experience across academia, non-profit, federal health consulting, and now pharma. She has worked in areas of informatics and platform development, including involvement with i2b2 implementation and OHDSI, and has published in areas of public health leveraging real-world evidence platforms. Leanne has a doctorate in public health focused on biostatistics and epidemiology from UCLA.