FIRST ANNOUNCMENT – SAVE THE DATE

BBS course

Does This Treatment Cause That Outcome: A Practical Guide to Estimand Thinking

Speaker: **Stephen J Ruberg**, PhD

Wednesday March 25th, 2026 (9:00-12:30)

In person only Biozentrum, Lecture hall U1.141, Basel
Costs: 200 CHF for Industry and 100 CHF for Academics/Students

Organizers: Fred Sorenson (Cencora), Charlene Mere (BMS), Lilla Di Scala (J&J)

Learning Objective: The ultimate objective is to help clinical trial practitioners (statisticians and clinicians) simplify and clarify the estimand framework so that they match important clinical questions/objectives with study design and analysis approaches and subsequent interpretation of study results. This will allow thoughtful engagement with all relevant stakeholders in a meaningful way. The course is based on the forthcoming book by Dr. Ruberg: *Does This Treatment Cause That Outcome? The Science of Estimating a Treatment Effect and Why It Matters.*

Speaker background: Stephen Ruberg has been in the pharmaceutical drug development arena for nearly 45 years with direct senior-level experience in several pharmaceutical companies and more recently as a consultant to many pharma companies. He has authored many publications on the topic of estimands and also been an invited speaker at many statistical conferences (as well as a couple of clinical conferences) to present on the topic of estimands. He also taught a short course at the 2024 and 2025 ASA Biopharmaceutical Section RISW conferences. He has lectured inside many pharmaceutical companies on the topic of estimands during his consulting career and has extensive teaching experience (all the way back to an undergraduate degree in education as well as mathematics).

Course background: Since the release of ICH E9(R1), the implementation of the estimand framework has received considerable attention. Despite this well-worn topic, based on presentations at recent statistical conferences and publications in the statistical literature, there are still considerable misunderstandings, differences of opinions, and outright misconceptions about estimands. This course will also highlight how some efforts have been unnecessarily complicated and confusing, thereby alienating clinical and other non-statistical collaborators in the clinical drug development process. This course emphasizes principles and creates clear and actionable considerations for creating meaningful estimands in clinical drug development.
The course will highlight how ICH E9(R1) is an attempt to clarify the definition of the treatment effect when there is incomplete data on the randomized study treatment. The four attributes will be examined carefully, but none more carefully than the definition of the study treatment. But it is also important to define what is meant by "effect." Two fundamental "effect questions" are posited: (1) what is the effect of initiating a treatment, and (2) what is the effect of taking a treatment? With treatment and the treatment effect question well-defined, many issues related to intercurrent events and the five strategies discussed in ICH E9(R1) become much more clear and simple. The Tripartite Estimand Approach (TEA) will also be discussed in detail as another approach that should be given strong consideration in many situations. Concepts from causal inference and the use of potential outcomes will underpin parts of the course. Examples across many disease states will be given throughout the lecture – e.g. oncology, immunology, diabetes, and neurodegeneration.

Course Outline (9am-12:30am)

Part 1 Some History
- The Goal of Science
- How Did We Get Here?
- The Advent of the ICH E9(R1) Addendum on Estimands

Part 2 ICH E9(R1) Explained
- The Four Attributes
- The Five Strategies
- The Tripartite Estimand Approach

Part 3 Weaving the Golden Thread
- Implementation of the Estimand Framework
- Examples of the Golden Thread

Part 4 Epistemology – What is the Truth?
- What Do We Mean by the Mean?
- Potential (Outcome) Problems