Course Title

Causal Inference in Randomized Controlled Trials: New Challenges and Perspectives

Category

Methodology

Target Audience

Statisticians working in industry, academia, government, and graduate students who are interested in causal inference and clinical trials.

Prerequisites for Participants

The course assumes basic familiarity with statistical inference. Prior knowledge of causal inference is not required.

Computer and Software Requirements

None

Course Description

Abstract

In drug development we usually aim to assess causal effects of investigational treatments compared to control treatments. Randomized controlled trials (RCT) have traditionally served as the basis for establishing these causal effects. While ICH E9 (R1) does not explicitly mention the word 'causal', the ideas around precisely defining treatment effects using potential outcomes (estimands) are closely related to causal inference. Indeed, oftentimes causal inference methods are needed to address intercurrent events (post-randomization events that affect either the interpretation or existence of outcomes). We believe that there is a need to raise awareness of the role of causal inference in RCTs and to prepare statisticians working on clinical trials with knowledge of causal inference tools appropriate to RCT settings.

This half-day short course introduces the basic concepts of causal inference and specific topics that are most relevant to RCTs. We start with a general introduction to causal inference including main assumptions and an overview of the role of causal inference in RCTs, followed by specific topics including estimation of causal effects, principal stratum estimands, and conditional and marginal treatment effects, with emphasis on their applications in RCTs. For each topic, the course content includes both theoretical knowledge/background and RCT examples.