Causal inference and AI/ML in pharmaceutical statistics

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Abstract

This short course introduces the basic concepts and fundamental methods of causal inference relevant to pharmaceutical statistics. Starting with the central questions in drug development and licensing and the roles of causal inference and AI/ML in answering them, the short course consists of three parts: (1) estimand framework, (2) efficient estimators, and (3) targeted learning. The short course covers causal thinking for different types of commonly used study designs in the pharmaceutical industry, including but not limited to randomized controlled clinical trials, single-arm clinical trials with external controls, and real-world evidence studies. The short course concludes with a case study along with a roadmap to conduct causal inference in clinical studies. The materials covered in this short course are extracted from the instructor's book, *Causal Inference in Pharmaceutical Statistics*, published by Chapman & Hall/CRC in 2024.