Robust inference of treatment effects in platform trials



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The platform trial is an innovative trial design that uses a master protocol (i.e., one overarching protocol) designed to evaluate multiple treatments in an ongoing manner and can expedite the development of potentially effective treatments. However, the flexibility that marks the potential of platform trials also creates inferential challenges. Key issues that are common to platform trials include (i) different eligibility criteria for different treatments; (ii) varying probabilities of treatment assignments; and (iii) concurrent and nonconcurrent controls. All these challenges can result in a lack of comparability among patients across different treatment arms, thereby introducing bias into a naïve comparison of treatments. In this project, we propose a rigorous framework for robust inference of treatment effects using concurrent controls. We first define an estimand that characterizes the treatment effect as the expected difference in potential outcomes under two treatments among concurrently eligible patients. Targeting this estimand, we will develop weighting and stratification methods for robust estimation without any modeling assumptions. We will also consider a model-assisted approach for covariate adjustment to gain efficiency without sacrificing robustness.

Thursday, March 7, 2024, 3:30-4:30PM Eastern

133 Rosenau Hall

Zoom Link:

https://unc.zoom.us/j/98423779288?pwd=b0tqYThCQTAxeDdTQ0FRY3RnazdwQT09

Meeting ID: 984 2377 9288

Passcode: 631794

