Statistics Seminar

Wednesday, September 17, 2014
4:15 pm
G01 Biotechnology

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Intention-to-Treat Analysis in Clinical Trials with Treatment Discontinuation and Missing Data

A recent National Research Council study on the prevention and treatment of missing data in clinical trials – see e.g. Little et al. (2012), New England Journal of Medicine, 367, 14, 1355-1360 – has received considerable attention. I discuss three analysis issues arising from that study. First, I distinguish treatment discontinuation from missing outcome data. Missing outcome data is a standard missing data problem, but treatment discontinuation is better viewed from the perspective of the causal literature on noncompliance. Second, the standard intention to treat (ITT) estimand, the average effect of randomization to treatment, is compared with three alternative estimands for the ITT population. I argue that one of these, a summary measure of the effect of treatment prior to discontinuation, merits more consideration. Third, I consider when follow-up measures after discontinuation are needed for valid measures of treatment effects. The answer depends on the choice of primary estimand, and the plausibility of assumptions needed to address the missing data. Ideas are motivated and illustrated by a reanalysis of a past study of inhaled insulin treatments for diabetes, sponsored by Eli Lilly. This is joint work with Shan Kang, a doctoral student at Michigan.

Refreshments will be served after the seminar in 1181 Comstock Hall.