

STATISTICS COLLOQUIUM

FRIDAY, MAY 2, 2014

TALK: 3:00 PM — SCIENCE CENTER RM. 705

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“A Principal Stratification Approach to Receipt of Rescue Medication in Early Escape Designs”

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ABSTRACT

Early escape designs were developed as a modification of placebo-controlled clinical trials in order to reduce the length of time sick subjects take an inactive treatment. In such designs, subjects that do not respond well to the assigned treatment receive additional rescue medication. Although early escape designs alleviate ethical concerns, they lead to complications with statistical analysis and interpretation. Comparisons between the group assigned to the active treatment and the group assigned to the placebo are no longer interpretable as comparisons of receipt of the active treatment versus receipt of the placebo because the treatment actually received may have changed from that assigned. Receipt of rescue medication is a post-treatment outcome that might also affect more clinically relevant outcomes. We approach this problem with the principal stratification framework, in which subjects belong to latent groups based on post-treatment variables, here, receipt of rescue medication. For one principal stratum, the estimands of causal effects are interpreted as the causal effect of receipt of active treatment versus placebo. We present the results from a placebo-controlled clinical trial of treatment for relapsing-remitting multiple sclerosis in order to motivate issues and demonstrate estimation of causal effects.