

Yale SCHOOL OF PUBLIC HEALTH

Biostatistics

Dealing with observed and unobserved effect moderators when estimating population average treatment effects

Elizabeth A. Stuart, PhD

**Professor and Associate Dean for Education
Department of Mental Health, Biostatistics and Health
Policy & Management
Johns Hopkins Bloomberg School of Public Health**

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ABSTRACT

Many decisions in public health and public policy require estimation of population average treatment effects, including questions of cost effectiveness or when deciding whether to implement a screening program across a population. While randomized trials are seen as the gold standard for (internally valid) causal effects, they do not always yield accurate inferences regarding population effects. In particular, in the presence of treatment effect heterogeneity, the average treatment effect (ATE) in a randomized controlled trial (RCT) may differ from the average effect of the same treatment if applied to a target population of interest. If all treatment effect moderators are observed in the RCT and in a dataset representing the target population, then we can obtain an estimate for the target population ATE by adjusting for the difference in the distribution of the moderators between the two samples. However, that is often an unrealistic assumption in practice. This talk will discuss methods for generalizing treatment effects under that assumption, as well as sensitivity analyses for two situations: (1) where we cannot adjust for a specific moderator observed in the RCT because we do not observe it in the target population; and (2) where we are concerned that the treatment effect may be moderated by factors not observed even in the RCT. These sensitivity analyses are particularly crucial given the often limited data available from trials and on the population. The methods are applied to examples in drug abuse treatment. Implications for study design and analyses are also discussed, when interest is in a target population ATE.