

Seminar Series: Myra Samuels Memorial Lecture

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YouTube livestream: https://www.youtube.com/channel/UC-u5x4FV67BnpBGigKHBeXg

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

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.

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Elizabeth A. Stuart, Ph.D., is Bloomberg Professor of American Health in the Department of Mental Health at the Johns Hopkins Bloomberg School of Public Health, with joint appointments in the Department of Biostatistics and the Department of Health Policy and Management. She received her Ph.D. in Statistics in 2004 from Harvard University and is a Fellow of the American Statistical Association (ASA) and the American Association for the Advancement of Science (AAAS). Professor Stuart has extensive

experience in developing methods for estimating causal effects and addressing the complications of missing data in experimental and non-experimental studies, particularly as applied to mental health, public policy, and education. Her primary research interests include designs for estimating causal effects in non-experimental settings (such as propensity scores), methods to assess and enhance the generalizability of randomized trials to target populations, and methods for policy evaluation. She has received research funding for her work from the National Science Foundation, the Institute of Education Sciences, the WT Grant Foundation, and the National Institutes of Health and has served on advisory panels for the National Academy of Sciences, the US Department of Education, and the Patient Centered Outcomes Research Institute. Professor Stuart received the Mid-Career Award from the Health Policy Statistics Section of the ASA, the Gertrude Cox Award for applied statistics, Harvard University's Myrto Lefkopoulou Award for excellence in Biostatistics, and the inaugural Society for Epidemiologic Research Marshall Joffe Epidemiologic Methods Award.



Myra Samuels (1940-1992)

The **Myra Samuels Memorial Lecture** is named in memory of Myra Samuels, who was Associate Professor of Biostatistics and Epidemiology in Purdue University's Department of Veterinary Pathobiology and Associate Director of Statistical Consulting in the Department of Statistics. She received her PhD in Statistics from the University of California, Berkeley, under Jerzy Neyman, and taught at

Purdue University for 24 years. Her research was oriented toward issues in biostatistics and included both conceptual issues in mathematical statistics and collaborations on applications. Professor Samuels was a member of the American Statistical Association, the Biometric Society and the Society for Clinical Trials. Her textbook, *Statistics for the Life Sciences*, first published in 1989, is now in its fifth edition, revised by J. Wittmer and A. Schaffner. The textbook is widely used in statistics courses.