Combining experimental and population data
to estimate population treatment effects

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With increasing attention being paid to the relevance of studies for real-world practice (such as especially in comparative effectiveness research), there is also growing interest in external validity and assessing whether the results seen in randomized trials would hold in target populations. While randomized trials yield unbiased estimates of the effects of interventions in the sample of individuals in the trial, they do not necessarily inform about what the effects would be in some other, potentially somewhat different, population. While there has been increasing discussion of this limitation of traditional trials, relatively little statistical work has been done developing methods to assess or enhance the external validity of randomized trial results. In addition, new “big data” resources offer the opportunity to utilize data on broad target populations. This talk will discuss design and analysis methods for assessing and increasing external validity, as well as general issues that need to be considered when thinking about external validity. The primary analysis approach discussed will be a reweighting approach that equates the sample and target population on a set of observed characteristics. Underlying assumptions and methods to assess robustness to violation of those assumptions will be discussed. Implications for how future studies should be designed in order to enhance the ability to assess generalizability will also be discussed.

Biography:
Elizabeth Stuart, Ph.D. is Vice Dean for Education and Bloomberg Professor of American Health in the Departments of Mental Health, Biostatistics, and Health Policy and Management at the Johns Hopkins Bloomberg School of Public Health. She received her Ph.D. in statistics in 2004 from Harvard University. Her primary research interests include the trade-offs in different study designs for estimating causal effects, and the use of causal inference methods in public health, mental health, and education.