



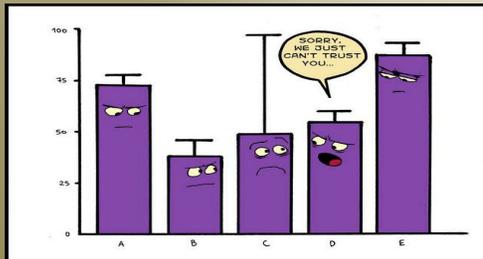
## NEW JERSEY CHAPTER

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**Friday, June 8, 2012**

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## Program

## Distinguished Speakers: Abstracts

### Zhiqiang Tan, Ph.D., Associate Professor, Department of Statistics, Rutgers, The State University of New Jersey.

**Abstract:** In this talk, we first review statistical methods for causal inference in cross-sectional studies. Such methods can depend on an outcome regression model, or a propensity score model, or even both to achieve double robustness against possible model misspecification. For illustration, we discuss an example on right heart catheterization for patients in intensive care units. Then we present new statistical methods for causal inference in longitudinal studies. A central idea is to consider average treatment effects conditional on realized treatment regimes over time, instead of hypothetical treatment regimes. This allows a simple and transparent generalization of the cross-sectional methods, including outcome regression and propensity score methods. We develop these methods in the context of studying antipsychotic treatment for elderly nursing home residents.

### Kosuke Imai, Ph.D., Assistant Professor, Department of Politics, Princeton University.

**Abstract:** The propensity score plays a central role in a variety of settings for causal inference. In particular, matching and weighting methods based on the estimated propensity score have become increasingly common in observational studies. Despite their popularity and theoretical appeal, the main practical difficulty of these methods is that the propensity score must be estimated. Researchers have found that slight misspecification of the propensity score model can result in substantial bias of estimated treatment effects. In this paper, we introduce covariate balancing propensity score (CBPS) estimation, which simultaneously optimizes the covariate balance and the prediction of treatment assignment. We exploit the dual characteristics of the propensity score as a covariate balancing score and the conditional probability of treatment assignment and estimate the CBPS within the generalized method of moments or empirical likelihood framework. We find that the CBPS dramatically improves the poor empirical performance of propensity score matching and weighting methods reported in the literature. We also show that the CBPS can be extended to a number of other important settings, including the estimation of generalized propensity score for non-binary treatments, causal inference in longitudinal settings, and the generalization of experimental and instrumental variable estimates to a target population.

### Michael Sobel, Ph.D., Professor, Department of Sociology, Columbia University.

**Abstract:** Randomized experiments are the gold standard for evaluating proposed treatments. The intent to treat estimand (ITT) measures the effect of treatment assignment, but not the effect of treatment if subjects take treatments to which they are not assigned. The desire to estimate the efficacy of the treatment in this case has been the impetus for a substantial literature on compliance over the last 15 years. In papers dealing with this issue, it is typically assumed there are different types of subjects, for example, those who will follow treatment assignment (compliers), and those who will always take a particular treatment irrespective of treatment assignment. The estimands of primary interest are the complier proportion and the complier average treatment effect (CACE). To estimate CACE, researchers have used various methods, for example, instrumental variables and parametric mixture models, treating compliers as a single class. However, it is often unreasonable to believe all compliers will be affected. This paper therefore treats compliers as a mixture of two types, those belonging to a zero effect class, others to an effect class. Second, in most experiments, some subjects drop out or simply do not report the value of the outcome variable, and the failure to take into

account missing data can lead to biased estimates of treatment effects. Recent work on compliance in randomized experiments has addressed this issue by assuming missing data are missing at random or latently ignorable. We extend this work to the case where compliers are a mixture of types and also examine alternative types of non-ignorable missing data assumptions. Key words: Causal Inference; Compliance; Latent Ignorability; Missing Data; Mixture Distributions

### Dylan Small, Ph.D., Associate Professor, Department of Statistics, The Wharton School, University of Pennsylvania.

**Abstract:** We will discuss two ways in which instrumental variables which are only partially valid may still be useful in making causal inferences. A valid instrument manipulates a treatment that it does not entirely control, but the instrument affects the outcome only indirectly through its manipulation of the treatment. The idealized prototype is the randomized encouragement design: subjects are randomly assigned to receive either encouragement to accept the treatment or no such encouragement, but not all subjects comply by doing what they are encouraged to do, and the situation is such that only the treatment itself, not disregarded encouragement alone, can affect the outcome. An instrument is weak if it has only a slight impact on acceptance of the treatment, that is, if most people disregard encouragement to accept the treatment. Typical applications of instrumental variables are not ideal: encouragement is not randomized, though it may be assigned in a far less biased manner than the treatment itself. Using the concept of design sensitivity, we study the sensitivity of instrumental variable analyses to departures from the ideal of random assignment of encouragement, with particular reference to the strength of the instrument. We find that no matter how large the sample size becomes, even if the effect under study is quite large, studies with weak instruments are extremely sensitive to tiny biases, whereas studies with stronger instruments can be insensitive to moderate biases. Another way in which a potential instrument can be invalid besides its being not randomized is that the instrument may influence both the treatment of interest and a secondary treatment also influenced by the primary treatment. This creates a violation of the exclusion restriction. We discuss extensions of instrumental variable methods that can be used for this setting. The two papers that will be discussed in this talk are joint works with Paul Rosenbaum and with Marshall Joffe, Tom Ten Have, Steve Brunelli and Harold Feldman.

### Miguel Hernan, Dr.P.H., Professor of Epidemiology, Department of Epidemiology, Harvard School of Public Health.

**Abstract:** Ideally, randomized trials would be used to compare the long-term effectiveness of dynamic treatment regimes on clinically relevant outcomes. However, because randomized trials are not always feasible or timely, we often must rely on observational data to compare dynamic treatment regimes. An example of a dynamic treatment regime is “start combined antiretroviral therapy (cART) within 6 months of CD4 cell count first dropping below  $x$  cells/mm<sup>3</sup> or diagnosis of an AIDS-defining illness, whichever happens first” where  $x$  can take values between 200 and 500. Inverse probability (IP) weighting of dynamic marginal structural models and the parametric g-formula can be used to estimate the  $x$  that minimizes 5-year mortality risk using observational data. Unlike standard methods, IP weighting and the g-formula can appropriately adjust for measured time-varying confounders (e.g., CD4 cell count, viral load) that are affected by prior treatment. Estimators based on the parametric g-formula are more efficient than IP weighted estimators. This is often at the expense of more parametric assumptions. Here we describe how to use the parametric g-formula to estimate risk by the end of a user-specified follow-up period under dynamic treatment regimes. We describe an application of this method to answer the “when to start” question using data from the HIV-CAUSAL Collaboration.

8:00-8:55	Registration and Breakfast
8:55-9:00	Opening Remarks <b>Prof. Dirk Moore, President, ASA-NJ Chapter</b>
9:00-10:05	Exploring New Statistical Methods for Causal Inference in Longitudinal Studies Zhiqiang Tan, Ph.D.
10:05-11:10	Covariate Balancing Propensity Score Kosuke Imai, Ph.D.
11:10-11:25	Break
11:25-12:30	Compliance Mixture Modeling with a Zero Effect Complier Class and Missing Data Michael Sobel, Ph.D.
12:30-1:45	Lunch
1:45-2:50	Strategies for Using Partially Valid Instrumental Variables Dylan Small, Ph.D.
2:50-3:05	Break
3:05-4:10	Comparative Effectiveness of Dynamic Treatment Regimes: An Application of the Parametric g-Formula Miguel Hernan, Dr. P.H.
4:10-4:55	Questions and Answers
4:55-5:00	Closing Remarks <b>Steve Ascher, Vice-President, ASA-NJ Chapter</b>