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A Principal Stratification M-Estimator using Principal Scores

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Abstract Text:

In a randomized controlled trial (RCT), some subjects assigned to the treatment condition may not fully comply. Often there is interest in the effect of the treatment within the "principal stratum" of subjects who would comply if assigned to treatment. However, it is unknown which control subjects would have complied if treated and which wouldn't. (This talk will be limited to the case of "one-sided" non-compliance, in which compliance is binary, so that principal stratum membership is observed in one treatment group and unobserved in the other.) One approach to identifying potential compliers in the control group uses "principal scores," probabilities of compliance, conditional on covariates, or $e(\boldsymbol{x}_i)= Pr(S_{Ti}=1|\boldsymbol{x}_i)$, where $S_{Ti}=1$ if subject i complies in the treatment condition and $S_{Ti}=0$ otherwise. To use principal scores, the analyst will typically either fit a parametric finite mixture model--for instance, if outcomes are continuous, assuming that $p(Y_{Ci} \mid e(\boldsymbol{x}_i))= e(\boldsymbol{x}_i)\varphi\{(x-\mu_1)/\sigma_1\}+(1-e(\boldsymbol{x}_i))\varphi\{(x-\mu_2)/\sigma_2\}$, where $p(Y_{Ci} \mid e(\boldsymbol{x}_i))$ is the density of i's control potential outcome conditional on i's principal score, φ is the standard normal density, and μ_1, μ_2, σ_1, and σ_2 are means and standard deviations to be estimated, or assume "principal ignorability," (c.f. Feller et al. [2017] and Ding and Lu [2017]) that potential outcomes are independent of principal stratum conditional on covariates, $Y_C \perp S_T|\boldsymbol{x}$. The mixture model approach is problematic if the distributional assumptions do not hold, and may be biased even when the model is correctly specified (Feller, et al. 2016). The approach based on principal ignorability is problematic if researchers do not believe the principal ignorability assumption, or if primary interest is in the difference between effects in different principal stratum.

In this talk I will introduce a semiparametric M-estimation approach based an an alternative assumption of no interactions between principal strata and

covariates in an outcome regression--specifically, given a model for Y_C conditional on covariates $\mathbf{x}$, with parameter vector $\boldsymbol{\beta}$, $f(\mathbf{x},\boldsymbol{\beta})$, $\mathbf{E}[Y_C - f(\mathbf{x},\boldsymbol{\beta})|S_T, \mathbf{x}]=\mathbf{E}[Y_C - f(\mathbf{x},\boldsymbol{\beta})|S_T]$. The estimator also assumes that estimated principal scores are unbiased. Given these assumptions, I will present a set of stacked estimating equations to estimate principal effects in the one-sided non-compliance case. Unlike typical mixture modeling, this new approach makes no distributional assumptions about potential outcomes.

I will present a simulation study showing that confidence intervals from the M-estimation approach achieve their nominal levels in several circumstances in which confidence intervals from (even well-specified) mixture models do not, and that the M-estimator is typically less biased than the mixture-modeling estimator, though the M-estimator tends to have higher standard errors. The root-mean-squared-error of the M-estimator improves absolutely and relative to the mixture modeling estimator as sample size increases and the relationship between $\boldsymbol{x}$ and S_T becomes stronger.

I will illustrate the M-estimator in a study of feedback methods within an intelligent tutoring system. Some students requesting hints within the software were randomly assigned to receive hints in video format, and others were randomly assigned to text hints. Computer log data shows that some students assigned to video did not watch the entire video. I will estimate the effect of assignment to video hints separately for the principal stratum of students who, if assigned to video hints, would watch the entire video, and the principal stratum of students who would not watch the entire video.

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