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"Beyond Intention to Treat: Efficient Estimation of Causal Effects in Randomized Trials with Noncompliance"

In randomized trials with non-compliance, estimating the causal effect of actually receiving the treatment compared to receiving the control is often of interest. Analytic approaches for such trials include intention-to-treat (ITT), as-treated (AT) and instrumental variables (IV) analyses. ITT analysis compares averages outcomes by randomization assignment regardless of the treatment received, and does not estimate the effect of actually receiving the treatment but instead the effect of assignment to the treatment group. AT analysis, which compares average outcomes by actual treatment received regardless of assignment, could be biased because it compares self-selected rather than randomized groups. Like AT, IV analysis aims to estimate the effect of actually receiving the treatment but does so in a way that compares randomized rather than self-selected groups. In this talk, I discuss motivation for going beyond ITT analysis to estimate the causal effect of actually receiving the treatment and review the assumptions and principles underlying IV analysis. I describe a new IV estimator that is more efficient than standard methods. Our estimator involves applying empirical likelihood with moment restrictions to mixture outcome distributions. I present simulations that demonstrate the gains provided by our new estimator and apply our method to data from a randomized trial of an encouragement intervention to improve adherence to prescribed depression treatments among depressed elderly patients in primary care practices.