Children's Hospital of Philadelphia PolicyLab

Propensity Score Methods for Analyzing Observational Data Like Randomized Experiments: Challenges and Solutions for Rare Outcomes and Exposures

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Randomized controlled trials are the “gold standard” for estimating the causal effects of treatments. However, it is often not feasible to conduct such a trial because of ethical concerns or budgetary constraints. We expand upon an approach to the analysis of observational data sets that mimics a sequence of randomized studies by implementing propensity score models within each trial to achieve covariate balance, using weighting and matching. The methods are illustrated using data from a safety study of the relationship between second-generation antipsychotics and type 2 diabetes (outcome) in Medicaid-insured children aged 10–18 years across the United States from 2003 to 2007. Challenges in this dataset include a rare outcome, a rare exposure, substantial and important differences between exposure groups, and a very large sample size.

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