Randomized trials and their observational emulations: a framework for benchmarking and joint analysis

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Abstract

A randomized trial and an observational study designed to emulate the trial select participants separately, but have the same eligibility criteria, collect information on the same baseline covariates, and compare the effects of the same treatments on the same outcomes. Treatment effect estimates from the trial and its emulation can be compared to benchmark observational analysis methods. In a simplified setting with complete adherence to the assigned treatment strategy and no loss-to-follow-up, we use graphical causal models to show how benchmarking exploits an exchangeability assumption between the populations underlying the trial and its emulation, to account for differences in the distribution of covariates between them. When this exchangeability assumption holds, and the usual conditions needed for the estimates from the trial and its emulation to have a causal interpretation also hold, we derive restrictions on the law of the observed data. If the data are not incompatible with these restrictions, we show that joint analysis of the trial and emulation data to estimate causal quantities in the population underlying the emulation is possible.