

Principal Stratification for Causal Inference in Experiments with Extended Partial Compliance

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Many double-blind placebo-controlled randomized experiments with active drugs suffer from complications beyond simple noncompliance. First, the compliance with assigned dose is often partial, with patients taking only part of their assigned dose whether active or placebo. Second, the blinding may be imperfect in the sense that there may be detectable positive or negative side effects of the active drug, and consequently single measures of compliance have to be extended to allow different compliance to active drug and to placebo. Efron and Feldman (1991, JASA) presented an analysis of such a situation and discussed inference for data response from the non-conditional data in the active treatment stem, which stimulated much discussion, including concerning the role of the intention-to-treat principle in such studies. Here, we formulate the problem within the “principal stratification” framework of Frangakis and Rubin (2002, Biometrics), which adheres to the intention-to-treat principle, and we present a new analysis of the Efron-Feldman data within this framework. Moreover, we describe precise assumptions under which dose-response can be inferred from such nonrandomized data, which may seem debatable in the setting of this example; otherwise, the results are compelling, at least to the authors. Although this talk deals in detail with the Efron-Feldman data, the same framework can be applied to many problems in both actual scenarios and social scenarios.

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with extended partial compliance
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Many double-blind placebo-controlled randomized experiments with active drugs suffer from compliance beyond simple non-compliance. First, the compliance with assigned dose is often partial, with patients taking only part of their assigned dose, either active or placebo. Second, the blinding may be imperfect in the sense that those may be detectable positive or negative side effects of the active drug, and consequently single measures of compliance have to be extended to allow different compliances to the drug and to placebo.

Stefan and Feldman (1996, JASA) presented an analysis of such a situation and discussed data in the entire treatment arm, which stratified much discussion, including concerning the role of the intention-to-treat principle in such studies. Here, we formulate the problem within the "principal stratification" framework of Fraydoun and Rubin (2002, Biometrics), which addresses to the intention-to-treat principle, and we present a new analysis of the Stefan-Feldman data within this framework. Moreover, we describe precise assumptions under which dose-related can be inferred from such non-compliance data, which may seem debatable in the setting of this example; however, the results are compelling, at least to the authors. Although this talk deals in detail with only the Stefan-Feldman data, the same framework can be applied to many problems in both actual settings as social sciences.

Stefan