Yale SCHOOL OF PUBLIC HEALTH
Biostatistics

SEMINAR

Cumulative Incidence Function under Two-Stage Randomization

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Abstract

In recent years personalized medicine and dynamic treatment regimes have drawn considerable attention. Dynamic treatment regimes are sets of rules that govern the treatment of subjects depending on their intermediate responses or covariates. Two-stage randomization is a useful set-up to gather data for making inference on such regimes. Meanwhile, more and more practitioners become aware of competing-risk censoring for event type outcomes, where subjects in a study are exposed to more than one possible failure and the specific event of interest may be dependently censored by the occurrence of competing events. We aim to compare several treatment regimes from a two-stage randomized trial on survival outcomes that are subject to competing-risk censoring. With the presence of competing risks, cumulative incidence function (CIF) has been widely used to quantify the cumulative probability of occurrence of the target event by a specific time point. However, if we only use the data from those subjects who have followed a specific treatment regime to estimate the CIF, the resulting naive estimator may be biased. Hence, we propose alternative non-parametric estimators for the CIF using inverse weighting, and provide inference procedures based on the asymptotic linear representation. In addition, test procedures are developed to compare the CIFs from two different treatment regimes. Through simulation we show the practicality and advantages of the proposed estimators compared to the naive estimator. Since dynamic treatment regimes are widely used in treating cancer, AIDS, psychological disorders and other illnesses that require complex treatment and competing-risk censoring is common in studies with multiple endpoints, the proposed methods provide useful inferential tools to analyze such data and will help advocate research in personalized medicine.

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