Use of Multistate Model for Multiple Endpoints in Oncology Clinical Trials Analysis and Designs

Chen Hu

Johns Hopkins University
E-mail: chu22@jhmi.edu

Abstract: In oncology clinical trials, disease progression and mortality are typically captured through a series of sequentially observed events, such as cancer recurrence and deaths. The relationship between covariate (e.g., therapeutic intervention and prognostic markers), recurrence, and death is often of interest, as it may provide key insights of optimal treatment decisions and future study designs. Analysis of these multiple endpoints however can be complicated due to censoring, under-reporting of intermediate event and potential correlation between events. We focus on how multistate model framework and semiparametric regression model can be used to handle these challenges and provide insights on evaluating treatment effect, identifying potential prognostic and predictive covariates on disease progression and mortality, and facilitate late-phase clinical trial designs. Asymptotic results and numerical examples based on Monte Carlo simulations and real trial data are presented.