Estimation of Treatment Effect for Survival Endpoint in Ongoing Trials without Unblinding

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Abstract

Many assumptions including the assumption regarding treatment effect are made at the design stage in protocol for power and sample size calculations in clinical trials. It is certainly desirable to check these assumptions during the trial using real data without unblinding. Methods for sample size re-estimation based on blinded data analyses have been proposed for normal and binary endpoints. In this paper, we consider the case of survival endpoint. Three procedures including a classification method and two EM algorithms are proposed for treatment effect estimation in ongoing trials without unblinding. Two of the methods incorporate information of a surrogate endpoint in the blinded analysis. Simulations are used to assess the performances of the procedures under different survival model settings. An example is used to illustrate the applications of the procedures.