Optimal designs for trials with discrete time survival endpoints

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ABSTRACT: In studies on event occurrence the main question is whether and when a certain event occurs. Examples of events are smoking initiation, premature termination of therapy and recovery from some disease. The timing of such events cannot always be measured very precisely and coarse time intervals are used instead. At the end of each interval the occurrence of the event is measured, until and including the interval the subject experiences the event, drops out from the study, or the study concludes. Discrete time survival data can be analyzed by using a generalized linear model that is a compilation of models for each time interval. Until recently, no research had been done on the optimal design of trials with discrete time survival endpoints. Design questions are the optimal number of time periods, the optimal number of subjects and the optimal allocation of subjects to the control and experimental condition. The optimal designs are derived under the condition that the budget for sampling and treating subjects and taking measurements is not exceeded. It is shown that no general guidelines on the design of trials with discrete time survival endpoints can be given. The optimal design depends on the underlying survival function and direction and size of the treatment effect. A computer program will be demonstrated that facilitates the calculation of the optimal design. Directions for future research will also be discussed.