

Optimal Design and Analysis in Phase II Basket Like Trials

Fang Liu

Merck

E-mail: fang.liu11@merck.com

Abstract: The primary goal of an exploratory oncology clinical trial is to identify an effective drug for further development. To account for tumor indication selection error, multiple tumor indications are often selected for simultaneous testing in a basket trial. In this article, we propose optimal and minimax two-stage basket trial designs for exploratory clinical trials. Inactive tumor indications are pruned in stage 1 and the active tumor indications are pooled at end of stage 2 to assess overall effectiveness of the test drug. The proposed designs explicitly control the type I and type II error rates with closed-form sample size formula. They can be viewed as a natural extension of Simon's optimal and minimax two-stage designs for single arm trials to multi-arm basket trials. A simulation study shows that the proposed design method has desirable operating characteristics as compared to other commonly used design methods for exploratory basket trials.