Bayesian Basket trial Design Accounting for Multiple Cutoffs of the Ambiguous Biomarker

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Abstract: Basket trial design enrolls patients with different cancer types but the same genetic mutation or biomarker to evaluate the treatment effect of a targeted therapy. However, the explicit biomarker sometimes may not be clearly identified. In this article, we propose a Bayesian basket trial design to account for multiple cutoffs of ambiguous biomarkers and select the optimal cutoff to maximize the benefit subpopulation. A two-stage design is proposed for the estimation. Secondly, we propose a simple method to cluster homogeneous subgroups within the families defined by the biomarker to enhance the power for detecting efficacious subgroups. Extensive simulations are conducted to demonstrate the operating characteristics of the two estimation methods in terms of probability of correct selection of optimal cutoff and probability of efficacy.