Adaptive two-stage design for two-arm in phase II clinical trials

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Abstract

In a two-arm phase II clinical trial, the purpose is to determine whether the experimental therapy is superior to the standard one. For ethical considerations, two-stage designs have been widely used. The sample size required in a two-stage design is determined by the expected difference in response rates between the two therapies under a specified significant level and power. In practice, however, it may be difficult to decide the expected difference, especially when there is no available information about the two therapies in the literature. In view of this, following the idea of Lin and Shih (2004), we propose an adaptive two-stage design, in which the expected difference in the alternative hypothesis can be flexibly changed at the second stage based on the outcome observed at the first stage. For the pre-specified significant level and power, the suitable design parameters are also derived according to the probability structure of the proposed design.

Keyword: two-arm phase II clinical trial, adaptive two-stage design, expected sample size, design parameters