

# Sample Size Determination Concerning Decision Making in Clinical Trials - Two Case Studies

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**Abstract:** A pivotal aspect of planning a clinical study is the calculation of the sample size. The calculation of an adequate sample size is crucial, a process by which we calculate the optimum number of participants required to be able to arrive at ethically and scientifically valid results. Generally, the sample size is a function of significance level, power, expected effect size, drop-out rate, allocation ratio, and the objective and design of the study. In reality, the sample size needs to take into account of the decisions to be made during the course of the study and at the end of the study. In many cases, the objective of the drug development is not only to exceed placebo, but also to identify a competitive drug candidate, especially for a disease area that the market is very crowded. Therefore, the sample size will not be simply based on significant p values, but be determined by quantitative decision criteria comparing to competing drugs. During the course of the study, interim analyses for futility are often performed. The sample size should be planned sufficiently so that the interim decision making is sound given various assumptions. At the end of phase 2, a decision to initiate phase 3 studies is usually made based on the predicted probability of success of which the size of the phase 2 trial is a key component. In this talk, two real examples will be given to illustrate these ideas for the sample size determination concerning decision making.