Term problem set

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R code used for computation is given at the end of the document.

Problem 1: (Preliminaries) For a randomized, placebo-controlled study of a statin for lowering LDL, we consider a reduction in LDL by 10mg=dL on average to be clinically meaningful, whereas patients in the placebo will experience no change on average. Assume that the variance of LDL is equal to 20mg=dL at both baseline and the 3-month follow-up in both groups.

a. What additional information or assumption(s) do you need to calculate a sample size?

b. State the assumptions you make and calculate the sample size required for a two-sided test at 5% significance.

Problem 2: (Go/no-go) In a two-stage trial of an experimental treatment with a planned futility interim analysis, 14 patients are first enrolled and treated in the first stage. An additional of 20 patients will be enrolled and treated if there is at least 1 response in the first stage. At the end of the trial, the treatment is deemed promising ("go") when there are at least 4 responses in all enrolled patients.

a. Suppose the true response rate is 5%. What is the expected value of the sample size?

For a true response rate of 5%, the expected sample size of the trial is 25 subjects.

b. Suppose the true response rate is 5%. Evaluate the probability of a "go" decision.

The probability of a "go" decision is given by

$$Pr(go) = Pr(S_{14} \geq 1, S_{34} \geq 4 | p = 0.05)$$
$$= ... = \Sigma_{n=1}^{14}[Pr(S_14 = n | p = 0.05) * \Sigma_{m=n}^{34}[Pr(S_{20} \geq 4 - m | p = 0.05)]].$$

For a true response rate of 5%, the probability of a "go" decision is 0.08.

c. Suppose the true response rate is 20%. Evaluate the probability of a "go" decision.

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## [1] 0.9041092
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Using the same formulation for calculating $Pr(go)$ as above, for a true response rate of 20%, the probability of making a "go" decision is 0.90.

d. Using your result in (b) as type I error rate and (c) as power, evaluate the sample size required for a fixed design with null response 5% and alternative response 20%.

[1] 50

Setting type I error to 0.08 and power to 0.90, we find that the sample size required for a fixed design is 50 subjects.

Problem 3: (Predictive distribution) Suppose X1 follows an exponential distribution with rate .

a. Give a conjugate prior for Derive its posterior distribution and the corresponding prior predictive distribution of X1.

b. Suppose X1 and X2 are exchangeable. Derive the posterior predictive distribution of X2 given X1.

c. Derive the posterior predictive distribution of $(X1 + X2)/2$ given X1.

R code