STAT 8004, Homework 4

Group # ... (Replace this)
Members: ... (Replace this)

Mar. 20, 2014

This homework is due Thu., 2014/03/27, 5:30pm.

Instructions: Generate a PDF file from it and submit the PDF file to blackboard. Each group should submit one file with file names **hw[number]-[groupnumber].pdf**. For exmaple, "hw01-1.pdf" for homeowrk 1 and group 1. Please also include your R code in the appendix.

Please use a calculator instead of statistical softwares to solve the problems.

Problem 1. (30 points) Thalidomide is a tranquilizer that was prescribed in the late 1950s and early 1960s to pregnant women, with the devastating result of over 12,000 birth defects in 48 countries, before it was banned in 1962 (it was never sold in the United States).

Recently, the drug has reappeared as a possible solution to a very different medical problem. The National Institutes of Health announced on October 31, 1995, the results of 30-hospital study of the effectiveness of thalidomide in healing mouth ulcers in AIDS patients. In the study, which was chaired by Dr. Jeffrey Jacobson of the Bronx Veteran Affairs Medical Center and the Mount SInai School of Medicine in New York, it was found that 14 of 23 patients who received thalidomide had their ulcers heal, compared with 1 of 22 patients who received a placebo. The researchers would like to know whether these results suggest that thalidomide is more effective at healing mouth ulcers than the placebo.

a). (10 points) Test $\mathrm{H}_0: \ OR = 1$ based on the Pearson Contingency Chi-square Test.

b). (10 points) Test $\mathrm{H}_0: \ OR = 1$ based on the Conditional Mantel-Haenszel Test.

e). (10 points)Test $\mathrm{H}_0: \ OR = 1$ based on the Cochran's Test.

Problem 2. (30 points) Consider the neuropathy clinical example we have discussed in class. In the study, there are two treatments, drug *vs.* placebo, where the response of interest is improvement in peripheral sensory perception. Previous studies suggest that the placebo control group probability of improvement is on the order of $\pi_2 = 0.20$. Now suppose that a total sample size of only $N = 400$ is feasible. Suppose the drug-placebo ratio is $r/(1-r) = 1$. With $\pi_2 = 0.2$, what level of power is there to detect

a). (10 points) a risk difference of 0.10?

b). (10 points) a relative risk of 1.5?

c). (10 points) an odds ratio of 2.0?

When doing this, note that as π_1 changes, so does π. For alternative hypothesis, you can assume that π_1 is always higher (the drug has better effect than placebo).

Problem 3. (30 points) You are interested in the sensitivity and specificity of a new biomarker to predict recurrence of ovarian cancer. A pathologist has reviewed sections of tumors stained with this biomarker and characterized the intensity of the staining. The data are recorded for each tumor as 0, +, ++ or +++. Of the 100 tumors examined 75 were 0, 11 were +, 4 were ++, and 10 were +++. The hypothesis is that the intensity of the staining can be used to predict recurrence of cancer. Your clinical collaborator then revealed which of the patients had had a recurrence of disease. There was 12% in the group coded 0; 18% in the + group; 25% in the ++ group and 80% in the +++ group. You can define various rules to classify people based on these data including (but not limited to) (1) those with no staining will not recur and those with any staining will or (2) only those that stain +++ will recur and those not +++ will not recur.

a). (10 points) What is the sensitivity and specificity for the two diagnostic rules (1) and (2) above?

b). (10 points) How would you advise your clinician to use a biomarker that had good specificity but had poor sensitivity?

c). (10 points) Calculate 95% confidence intervals for sensitivity and specificity for the diagnostic rule (2). Note that both sensitivity and specificity should be bounded by (0,1). (Hint: log-negative-log transformation)