BIOS 665: Problem Set 1

Assigned: Tuesday, August 29, 2017

Due: Tuesday, September 12, 2017

Note: For all hypothesis tests, please state the method, the null hypothesis, the test statistic, the p-value, and the interpretation of the test using a significance level of 0.05, unless otherwise stated.

Helpful hints: For estimates and tests, simply copying and pasting SAS output without any commentary will not earn full credit, especially on exams. Highlighting is not considered as commentary. However, commentary can be as simple as: ‘The 95% CI for the odds ratio is (\_\_\_, \_\_\_\_).’

1. A randomized study of 160 subjects was conducted to evaluate whether a test treatment had a better outcome than placebo, in which the outcome was either a favorable or unfavorable response. Of the 80 subjects in the test treatment group, 45 had a favorable response, while out of the 80 subjects in the placebo group, 29 subjects had a favorable response.
   1. Construct a contingency table summarizing the association between treatment assignment and response.
   2. Calculate an estimate of the proportion of subjects with a favorable response in each of the treatment groups along with corresponding two-sided 95% confidence intervals.
   3. Calculate an estimate for the difference in proportions and a corresponding two-sided 95% confidence interval comparing test treatment versus placebo.
   4. Conduct a statistical test for whether an association exists between treatment group and response. Please also list two conditions that the data must meet for this test to be valid.
   5. Briefly discuss your results from parts (b.), (c.), and (d.).
2. A multi-center randomized trial was conducted for comparing two doses of a test drug for management of a chronic medical disorder. A contingency table of all subjects classified by the dose (high treatment dose, low treatment dose) and outcome (favorable response, unfavorable response) is given below.

|  |  |  |
| --- | --- | --- |
| Treatment | Favorable  Response | Unfavorable  Response |
| High Dose | 45 | 14 |
| Low Dose | 22 | 33 |

* 1. Estimate the odds ratio for favorable response (vs. unfavorable) comparing high dose to low dose.
  2. Provide a two-sided 95% confidence interval for this odds ratio.
  3. Interpret (b.) in light of a statistical test for the association between dose group and response.
  4. The contingency table from one of the centers is given below.

|  |  |  |
| --- | --- | --- |
| Treatment | Favorable  Response | Unfavorable  Response |
| High Dose | 7 | 2 |
| Low Dose | 3 | 4 |

* + 1. Calculate a corresponding two-sided 95% confidence interval for the odds ratio for favorable response (vs. unfavorable) comparing high dose to low dose for subjects in this center. Interpret the odds ratio estimate.
    2. Apply a two-sided statistical test. Justify your choice for the test.

The data shown below are from a study to evaluate the performance of a screening test for 130 patients for whom a particular disorder was known to be present and for 84 patients for whom it was known to be absent.

|  |  |  |
| --- | --- | --- |
| **Disease** | **Test+ Test-** | **Total** |
| Present | 106 24 | 130 |
| Absent | 26 58 | 84 |

* 1. Estimate the sensitivity and the specificity for the screening test.
  2. Provide a two-sided 99% confidence interval for sensitivity.
  3. Provide a two-sided 95% confidence interval for specificity.
  4. If 70% of the population has the disease and 30% does not, estimate the proportion of non-diseased patients among those who have a negative test.

1. A randomized study was undertaken to evaluate whether a new eye drop was effective at clearing proteins from the eye lens (*i.e.,* clearing proteins is the desired outcome). Each patient had the placebo drop placed in one eye that was chosen at random and the new treatment drop in the other eye. There were 260 patients enrolled with the results as follows:

|  |  |  |
| --- | --- | --- |
|  | **New Treatment** | |
| **Placebo** | Clear | Not Clear |
| Clear | 136 | 19 |
| Not Clear | 53 | 52 |

* 1. Use a statistical test to assess whether the probability of clearing proteins with the new drop is the same as the probability of clearing proteins with the placebo drop. Justify why you used this test.
  2. Is this result good news or bad news for the treatment drop?
  3. [For BIOS students and those trying for an H grade]: Create a two-sided 90% confidence interval for the difference in proportions of clearance between the new treatment and placebo.

1. Suppose a new clinical trial is being planned to confirm the findings of the study from Problem #2 (all patients). Suppose the expected probabilities of favorable response are 0.75 for high dose and 0.40 for low dose.
   1. With balanced allocation (i.e., equal sample size for each treatment group), determine the sample sizes needed to provide about 0.90 power at the two-sided 0.05 significance level for this study.
   2. With twice as many patients for high dose as for low dose, determine the sample sizes needed to provide about 0.80 power at the two-sided 0.01 significance level for this study with the same proportions as above.
   3. If at the end of the study there were 75 patients enrolled in each group, what is the power of the study if the proportions are the same as above, under equal allocation and a significance level of 0.05?