BIOS 665: Problem Set 1
Assigned: Sep 03, 2019
Due: Sep 12, 2019

Note: For all hypothesis tests, please state the statistical method you are using, the null hypothesis, the test statistic, the p-value, and the interpretation of the test using significance level of 0.05, unless otherwise stated. Please round all estimates except for p-values to three decimal places.

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 commentary. However, commentary can be as simple as: The 95% CI for the odds ratio is (___, ____).

1. A randomized study of 150 participants was conducted to evaluate whether a test treatment group had a better outcome than placebo group with the outcome being either favorable or unfavorable response. 38 of 75 participants in the test treatment group had a favorable response, while 23 of 75 participants in the placebo group had a favorable response.

   a) Construct a contingency table summarizing the association described between the assignment groups and the response.

   b) Calculate an estimate of the proportion of participants with favorable response in each of the treatment groups, along with corresponding two-sided 95% confidence intervals.

   c) Estimate the difference in proportions and provide a corresponding two-sided 95% confidence interval for comparing the test treatment and placebo groups.

   d) Conduct a statistical test for whether there exists any association between the treatment group and response. Please list at least two assumptions for this test to be valid.

   e) Interpret and briefly discuss your results from parts b)-d).

2. A two-center randomized clinical trial was conducted for comparing two doses of a test drug for management of a chronic eye irritation. A contingency table of all participants classified by center, the dose (high treatment dose, low treatment dose) and outcome (satisfactory response/unsatisfactory response) is given below.

Center	Treatment	Satisfactory Response	Unsatisfactory Response
A	High Dose	53	17
	Low Dose	23	35
B	High Dose	7	3
	Lose Dose	2	5

For center A only:

a) Estimate the odds ratio for satisfactory response (versus not), comparing high dose versus low dose.

b) Provide a two-sided 95% confidence interval for this odds ratio.

c) Interpret part b) in light of a statistical test for the association between dose group and response

d) For center B only:

i) Calculate a corresponding two-sided 95% confidence interval for the odds ratio for the comparison between high dose and low dose for satisfactory response or not for participants in Center B. Briefly interpret this odds ratio estimate.

ii) Apply a two-sided statistical test, and briefly interpret the results.

iii) Justify your choice of test in ii).

3. 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 placed in one eye chosen at random and the new treatment in the other eye. There were 240 patients enrolled with results as follows:

	New Treatment	
Placebo	Clear	Not clear
Clear	132	22
Not clear	53	33

a) 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. Justify your choice for selecting this test.

b) Is this result favorable or unfavorable for the new treatment drop relative to the placebo?

c) [For BIOS students and those trying for an H grade]. Construct a two-sided 90% CI for the difference in proportions of clearance between treatment and placebo.

4. The table below shows data from a study to evaluate the performance of a screening test for 130 patients for whom a particular disorder (cataract) was known to be present and for 70 patients for whom it was known to be absent.

Disease	Test +	Test -	Total
Present	106	24	130
Absent	22	48	70

   a) Estimate the sensitivity and the specificity for the screening test.

   b) Provide a two-sided 0.90 confidence interval for sensitivity.

   c) Provide a two-sided 0.99 confidence interval for specificity.

   d) If 70% of the population has the disease (i.e., prevalence) and 30% does not, estimate the proportion of the non-diseased patients among those who have negative test.

5. In designing a randomized clinical trial for the evaluation of a treatment for cardiovascular disease in terms of a favorable outcome after 6 months of follow-up, you expect favorable response rates of 0.67 for the test treatment and 0.43 for an appropriate control after this follow-up period.

   a) Using a two-sided 0.05 significance level with balanced allocation to these two groups, determine the sample size that would be necessary to provide 0.90 power for this planned study.

   b) With twice as many patients for the test treatment as for the appropriate control, 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 expected proportions of favorable response as above.

   c) If at the end of the study there were 83 patients enrolled in each group, what is the power of the study if the expected proportions of favorable response are the same as above, under balanced allocation and a two-sided significance level of 0.05?