Analysis of Categorical Data (BIOS 665) Mid-term Examination 2016

Exam Date: October 18, 2016

Take-home due date: October 27, 2016 at 11:00am

Requirements:

- For estimates, confidence intervals, and tests, simply copying and pasting SAS output without any commentary will not earn full credit. Highlighting is not considered commentary. However, commentary can be as simple as: "The 95% CI for the odds ratio is (____, ___)."
- For the take-home exam, **print each question on a separate page**, and **put your name on every page**. You should have 12 pages (1 signed face sheet, plus 1 per problem; you do not need to submit the description for Part IV). Do <u>not</u> staple the entire exam together, but do staple individual problems together if they span multiple pages. Bring your exam to class on the due date, where you will distribute each problem into the appropriate stack (one per problem). This will facilitate the grading process.
- Please note that p-values may be reported as ranges based on the table given below. For example, your answer may be '0.025 '. However, if using software, you should report p-values more precisely (such as 3 decimal places).
- In class exam: you may use one side of one 8.5 x 11" sheet of paper as a formula sheet. Please be sure your name is on your formula sheet, and submit this along with your exam.
- For each hypothesis test, provide the null hypothesis, test statistic, degrees of freedom, and conclusion.

| Honor Pledge: | I have neither | received nor | given | un authorized | aid on this e | xam. |
|---------------|----------------|--------------|-------|---------------|---------------|------|
| | | | | | | |

Chi-Square Distribution: Table of quantiles/critical values $(\chi^2_{df,1-\alpha})$

| df/α | 0.10 | 0.05 | 0.025 | 0.01 | 0.001 |
|------|------|-------|-------|-------|-------|
| 1 | 2.71 | 3.84 | 5.02 | 6.63 | 10.83 |
| 2 | 4.61 | 5.99 | 7.38 | 9.21 | 13.82 |
| 3 | 6.25 | 7.81 | 9.35 | 11.34 | 16.27 |
| 4 | 7.78 | 9.49 | 11.14 | 13.28 | 18.47 |
| 5 | 9.24 | 11.07 | 12.83 | 15.09 | 20.52 |

Z-scores: Quantiles/critical values $(Z_{1-\alpha/2})$

 $Z_{0.8}=0.842$, $Z_{0.9}=1.282$, $Z_{0.95}=1.645$, $Z_{0.975}=1.960$, $Z_{0.99}=2.326$, $Z_{0.999}=3.090$

Part I
Problems 1-5 are based on the contingency table shown below which is from a randomized,
multi-center, controlled clinical trial for the evaluation of a treatment for cardiovascular disease
in terms of a favorable outcome after 6 months of follow-up.

| Center | Treatment | Favorable | Unfavorable | Total |
|--------|-----------|-----------|-------------|-------|
| 1 | Test | 29 | 31 | 60 |
| 1 | Control | 24 | 36 | 60 |
| 1 | Total | 53 | 67 | 120 |
| 2 | Test | 40 | 20 | 60 |
| 2 | Control | 35 | 25 | 60 |
| 2 | Total | 75 | 45 | 120 |
| 3 | Test | 35 | 25 | 60 |
| 3 | Control | 17 | 43 | 60 |
| 3 | Total | 52 | 68 | 120 |

1. For Center 1, provide an estimate for the proportion of favorable outcome for each of the two treatment groups as well as a two-sided 95% confidence interval for each proportion.

2. For Center 1 only, and under minimal assumptions, assess whether there is a difference in the proportion of favorable outcome between the treatment groups using the two-sided 0.05 significance level. Briefly justify your method in 1-2 sentences.

3. Pooling across Centers 2 and 3 (and excluding Center 1), provide a two-sided 95% confidence interval for the difference between the probabilities of favorable outcome for those on test treatment versus control.

Hint: Combine Centers 2 and 3 for each of the cells in the 2x2 table.

4. Under minimal assumptions, and controlling for all three centers, assess whether there is an association between treatment and response. Briefly interpret your results in 1-2 sentences.

| Name: | |
|-------|--|
| _ | Calculate an estimate for the common odds ratio that accounts for stratification across the three centers. |

Part II

6. In designing a randomized study for the test treatment (versus a suitable control) in a population of patients with cardiovascular disease, you expect favorable response rates of 0.65 for the test treatment group and 0.50 for the control group. Using a two-sided 0.05 significance level with balanced allocation, determine the sample size <u>per group</u> that would be necessary to provide 90% power for this planned study.

Part III

For Problems 7 and 8, in a particular health-related study, a logistic model for the probability of favorable response (at a follow-up visit) was fit based on reference cell coding as expressed through the descriptions of the parameters in the table below. The results include selected parameter estimates, their standard errors, and the corresponding chi-square test statistics.

7. Fill in the missing values of the following table; present all results to three decimal places. Please show your calculations.

| Parameter | Estimate | SE | χ^2 | Odds Ratio | 95% CI for OR |
|--|----------|-------|----------|---------------|---------------|
| Intercept | -0.280 | | 2.110 | | |
| Region (East vs. West) | -0.530 | 0.268 | | | |
| Baseline Visit (Favorable vs. Unfavorable) | | 0.390 | 0.960 | | |
| Treatment (Test vs. Placebo) | 1.250 | 0.378 | | | |

Note: SE = standard error of the estimate; CI = confidence interval; OR = odds ratio

^{**}Hint: The estimate of baseline visit should be a positive value.

8. Provide two predicted probabilities of favorable response at follow-up (one for those on the test treatment and one for those on placebo) for patients in the east region with an unfavorable response at the baseline visit.

Part IV

For Problems 9 and 10, the table below shows data from a study in which 100 patients having a mild dermatological condition received test treatment on one of their arms and a placebo on the other arm. They were randomized as to which arm (left or right) received the test treatment. Data on their outcomes of clinical improvement on each arm after one week are recorded below.

| Clinical Improvement on Test | Clinical Improvement on Placebo | | Total |
|------------------------------------|---------------------------------------|----|-------|
| on Test | Yes | No | |
| Yes | 17 | 59 | 76 |
| No | 19 | 5 | 24 |
| Total | 36 | 64 | 100 |

9. Use a statistical test to assess whether the probability of clinical improvement for the test treatment is equal to the probability of clinical improvement for the placebo.

10. Provide a quantity which expresses the extent to which a patient has greater odds of clinical improvement on the test treatment than on control. Briefly interpret your results from Part IV in 1-2 sentences.

Part V

11. A health sciences research group is conducting a study on the factors that influence the uptake of a vaccination (i.e., receiving a 'flu shot') for children in the elementary schools in a particular community. The table shown below summarizes the findings from this study comparing male and female students with respect to their intention to receive the vaccination during the upcoming flu season.

| | | Response | | |
|--------|---------------------|---------------------|---------------------|-------|
| Gender | Definitely will not | Probably will | Definitely will | Total |
| | receive vaccination | receive vaccination | receive vaccination | |
| Female | 12 | 23 | 60 | 95 |
| Male | 18 | 32 | 55 | 105 |
| Total | 30 | 55 | 115 | 200 |

Under minimal assumptions, assess whether there is a location shift in the intention to receive vaccination between genders through an appropriate statistical test at the two-sided 0.05 level. In 2-3 sentences, interpret your findings and indicate which gender has a greater intention to receive the vaccine (if appropriate), as well as justifying your choice of scores.