Biostatistical Methods I (BIOS 5710) Breheny

Assignment 8 Due: Wednesday, November 5

- 1. You have developed a new, cholesterol-lowering drug as a treatment for patients with high cholesterol. From past studies, you know that the standard deviation of cholesterol levels for patients in the population you're looking at is about 30 mg/dl. You assume that your new drug will reduce cholesterol levels by about 10 mg/dl on average, and that the placebo will have no effect on patients' cholesterol levels.
 - (a) Suppose you carried out a two-sample study, randomizing separate patients to drug and placebo. What is the power of a two-sample *t*-test with n = 100 subjects in each group given the assumptions above?
 - (b) Suppose instead that you designed a crossover trial to test the efficacy of the drug. If a patient's cholesterol level on drug and a patient's cholesterol level on placebo were completely independent, what is the standard deviation of the difference between a patient's level on each treatment?
 - (c) Using the standard deviation you calculated in (b), what is the power of a paired *t*-test with n = 100 subjects?
 - (d) Comment on the difference between the power you calculated in (a) and in (c). In particular, which was the more powerful test, and what can you conclude from this comparison?
 - (e) Do you feel the assumption of independence in (b) is believable? Why or why not?
- 2. A researcher has developed a preventative therapy that she expects to cut the risk of diabetes in half. In the population she is researching, the current prevalence of diabetes is 20%.
 - (a) Suppose she carries out this test with n = 100 subjects in the treatment group and n = 100 subjects in the control group. What is her power to detect a difference?
 - (b) How many subjects does she need in each group in order to achieve at least 80% power?
- 3. Consider representing observations from a binomial distribution as a 1×2 contingency table, where one cell counts the success and the other cell counts the failures. Suppose we carried out a χ^2 test of $H_0: \pi = \pi_0$ on this data by calculating

$$X^{2} = \sum_{i=1}^{2} \frac{(O_{i} - E_{i})^{2}}{E_{i}}$$

and comparing it to a χ_1^2 distribution. Show that this test is identical to the z test we discussed in class (Oct. 6), in the sense that the two tests will always produce identical *p*-values.

4. Consider testing for a difference in proportions by treating proportions as means and using a centrallimit theorem-based approach. In particular, let $\hat{\pi}_1$ denote the sample proportion in group 1, $\hat{\pi}_2$ the sample proportion in group 2, and n_1 and n_2 the respective sample sizes. Then

$$\frac{\hat{\pi}_1 - \hat{\pi}_2 - \Delta}{\mathrm{SE}} \sim \mathrm{N}(0, 1)$$

for some Δ and SE.

- (a) What is Δ ? What does Δ equal under the null hypothesis of no difference between groups?
- (b) What is SE?
- (c) Consider estimating SE under the null by calculating $\hat{\pi}$, the overall proportion obtained by pooling both groups together, then plugging that value in for π_1 and π_2 in your expression for SE. Carry out this test for the Lister experiment we discussed in class.
- (d) Compare your answer to the result from the χ^2 -test we carried out in class and (briefly) discuss their relationship.