Principles of Inferential Statistics in Medicine

Final Exam – EPIB–607, December, 2002.

1.   A clinical trial examining a new treatment for the common cold has just been completed. A paired design was used, with each subject receiving the new treatment being paired with another subject who received a placebo. Within each pair, a decision is made as to whether the subject receiving the new treatment or the subject receiving the placebo has fewer symptoms on the second day following the onset of the cold. Out of 12 pairs participating in the trial, the treatment does better than placebo in ten pairs, and the placebo does better in the other two pairs.

Perform an appropriate one-sided exact procedure to examine if treatment leads to better outcomes than placebo. State the null and alternative hypotheses, show your calculations, and state your conclusion.

2.   Quality of life scores are often non-normally distributed in healthy populations, because many values tend to be near the upper range of the scale. The SF-36 scale ranges from 0 to 100, with 100 indicating perfect health. In a study measuring the quality of life of patients with wrist fractures, the following SF-36 data are collected:

Patients with wrist fractures	Healthy (unmatched) controls
85	98
87	93
97	99
62	89
75	65
92	100
91	
81	
76	
88	
90	

Perform an appropriate two-sided nonparametric test to examine if subjects with wrist fractures have different quality of life compared to healthy subjects. State the null and alternative hypotheses, show your calculations, and state your conclusion.

3.   Below is a triplot from a study estimating the probability that a new medication relieves arthritic pain within three hours. The data were such that

30 out of 70 patients experienced relief within three hours.
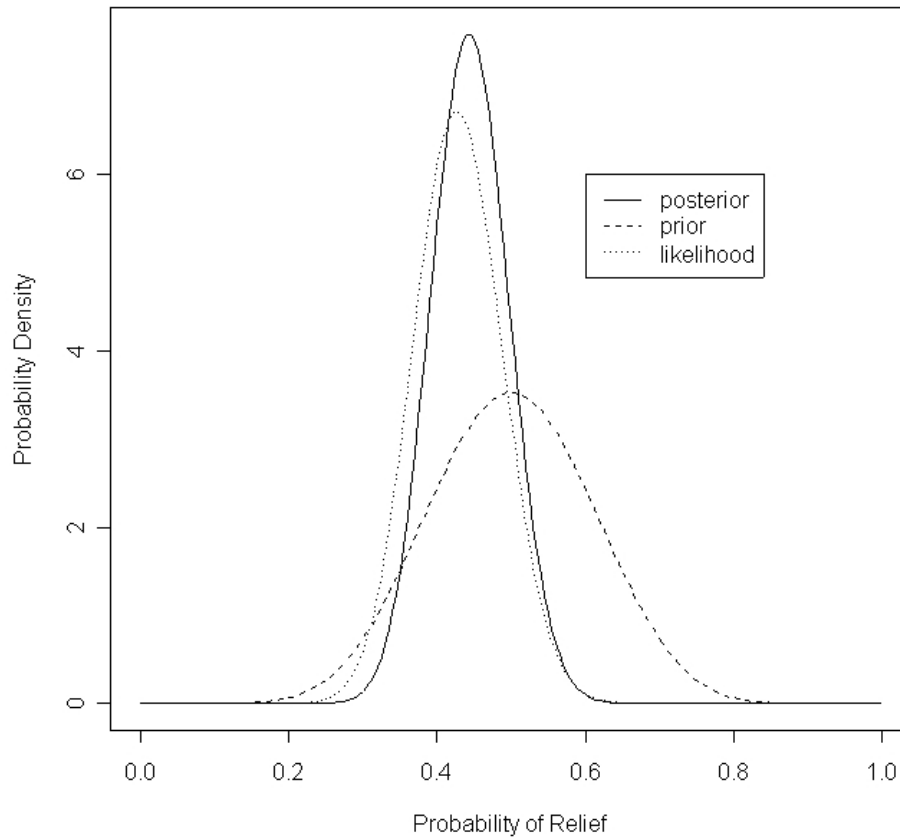
(a) Using the above data, provide a frequentist 95% confidence interval for the probability of relief within three hours.

(b) From the plot, provide an approximate 95% posterior credible interval.
(c) Compare your answers in (a) and (b) in terms of numeric values and interpretations.
(d) The prior distribution used was a beta. What would you guess the prior parameter values were?

4.   The scatterplot and best fitting regression line below is taken from a study of the relationship between age and quality of life. Quality of life was measured by the SF-36 scale, which ranges from 0 to 100 points.

(a) Provide a rough estimate of the slope and intercept of the regression line.
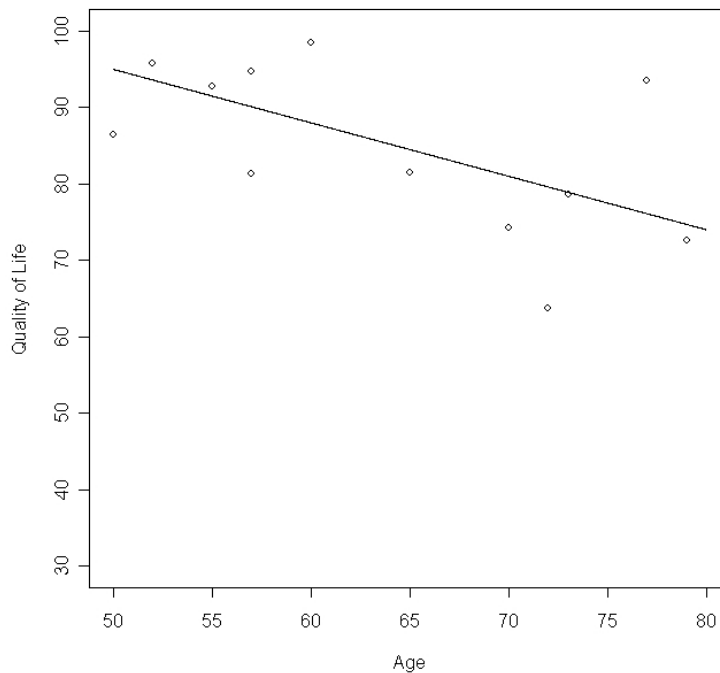
Provide interpretations of the meaning of these quantities in practice.

(b) Provide a rough (by eye) estimate of the value of the standard deviation about the regression line (residual error).

5.   The following data have been collected from one individual as part of a study on the stability of plasma ferritin levels over time (time is measured from the start of the study).

Weeks from start of study	plasma ferritin in mg/L
0	60
2	70
4	50
10	80

Calculate the intercept and slope of the least squares regression line for these data. Provide an interpretation for the slope of the line.

6.   A study is being designed to compare a new treatment for pain from osteoarthritis with a standard treatment. The standard therapy relieves pain 80% of the time, while the new treatment is expected to relieve pain 90% of the time, but will be considered a worthwhile alternative if it works 85% of the time or better. Assuming equal sample sizes in the two groups, what sample size would you recommend for each group? Explain your reasoning.

7.   There are two common side effects following a particular type of surgery, infection and nausea. 20% of all individuals are unlucky, and experience both of these side effects, while 40% experience nausea but not infection. What is the probability that a patient will experience infection, given that they already have nausea?

8.   An insurance company carries out a study which, in part, estimated the correlation coefficient between the age of married men and the age of their spouses. It is found that $r = 0.8$, that is, the estimated correlation coefficient between married partners was 0.8.

(a) Approximately what would the slope of the regression line be if the age of men (y variable) was regressed against the age of women (x variable)? Assume that the standard deviations of men and women are approximately equal.

(b) Again assume $r = 0.8$, but now suppose that the standard deviation in men was larger than that in women. Would the slope now be the same, larger, or smaller than the slope you approximated in part (a)?

9.   Label each statement as true or false, and explain why:

(a) A 99% confidence interval for a parameter μ is calculated, and is found to exclude zero. Therefore, a test of the null hypothesis $H_0 : \mu = 0$ versus $H_a : \mu > 0$ is necessarily significant at the 1% level.

(b) Let X follow a Poisson distribution with parameter $\mu = 2$. Then $Pr\{X \leq 2\} = 0.5$.

10.   In a pilot study of a new blood pressure lowering drug, a 95% confidence interval for the difference (before — after) in blood pressure is calculated, and found to be (4, 15), where the units are the usual $mm\ Hg$. An important difference is considered to be one that is 10 points or more.

(a) Provide a strict "technically correct" interpretation of this 95% confidence interval.

(b) Provide an interpretation that is not necessarily "technically correct", but is more useful in practice than the strict interpretation in part (a).

(c) Overall, what would you conclude about the effectiveness of this new drug, based on information in the pilot study?