Question 6

Intent of Question

The primary goals of this investigative task were to assess students’ ability to (1) evaluate whether a double-blind randomized experiment is possible, (2) construct and interpret a confidence interval for the difference between two proportions, (3) estimate a relative risk and construct and interpret a confidence interval for a relative risk, and (4) compare the confidence interval for the difference between two proportions and the confidence interval for the relative risk.

Solution

Part (a):

Yes, it can. An experiment is said to be double-blind when the control or comparison group and the treatment group are treated exactly alike, except for the treatments of interest, so that neither the patients nor the researchers who care for the patients and take measurements know which treatment has been assigned to any patient. Because treatment A must be administered by pill and treatment B must be administered by injection, then a placebo could be introduced for each treatment group as follows to make the study double-blind:

The patients who get treatment A also get a placebo injection. The patients who get treatment B also get a placebo pill. The physician does not have to know which treatment the patient is receiving if the treatment plus placebo is sent to him or her by a researcher not in contact with the patients (this researcher is the only one who knows which treatment each patient is receiving).

Part (b):

Step 1: Identify the appropriate confidence interval by name or formula and check appropriate conditions. (The question states that the conditions for inference have been met.)

Two-sample \( z \) confidence interval for the difference of two proportions.

OR

The formula for the confidence interval given in step 2 is provided.

Step 2: Correct mechanics.

Let \( \hat{p}_A = \frac{38}{154} = 0.2468 \equiv 0.25 \) and \( \hat{p}_B = \frac{16}{164} = 0.0976 \equiv 0.10 \). Then, the 95 percent confidence interval is

\[
(\hat{p}_A - \hat{p}_B) \pm z^* \sqrt{\frac{\hat{p}_A(1 - \hat{p}_A)}{n_A} + \frac{\hat{p}_B(1 - \hat{p}_B)}{n_B}} = \left( \frac{38}{154} - \frac{16}{164} \right) \pm 1.96 \sqrt{\frac{38}{154} \left( 1 - \frac{38}{154} \right) + \frac{16}{164} \left( 1 - \frac{16}{164} \right)}
\]

\[
= 0.1492 \pm 1.96 \times 0.0418
\]

\[
= 0.1492 \pm 0.0818
\]

\[
(0.0674, 0.2310)
\]
Step 3: Interpretation.

At the 95 percent confidence level, the range of plausible values for the true difference in the 15-year survival rates for the two treatments is 0.0674 to 0.2310. Because this interval is entirely above zero, this suggests that the 15-year survival rate is higher for treatment A.

Part (c):

The estimate of the relative risk is

$$\hat{\frac{p_A}{p_B}} = \frac{38}{154} = \frac{16}{164} = 2.5292 \approx 2.53$$

Part (d):

A 95 percent confidence interval for the relative risk is found by evaluating

$$e^{0.3868} \text{ to } e^{1.469},$$

which is 1.47 to 4.34.

At the 95 percent confidence level, the range of plausible values for the relative risk is 1.47 to 4.34. People are between 1.47 and 4.34 times more likely to survive 15 or more years with treatment A than with treatment B.

Part (e):

When the proportions of people who survive are low, as is the case with 0.25 and 0.10, it may be more meaningful or vivid to know that a patient’s chance of survival with treatment A is 1.47 to 4.34 times what it would be with treatment B rather than to know that the difference in the proportions of people who survive is 0.07 to 0.23, which does not sound like very much.

Scoring

This problem is scored in four sections. Section 1 consists of part (a). Section 2 consists of part (b). Section 3 consists of part (c) and part (d). Section 4 consists of part (e). Sections 1, 2, 3, and 4 are each scored as essentially correct (E), partially correct (P), or incorrect (I).

Section 1 [part (a)] is scored as follows:

Essentially correct (E) if the student (1) communicates what is meant by a double-blind study and (2) clearly describes how this experiment can be performed as a double-blind experiment.

Partially correct (P) if only one component is correct.

Incorrect (I) otherwise.
Section 2 [part (b)] is scored as follows:

Essentially correct (E) if the correct confidence interval is identified and constructed AND interpreted in context.

Partially correct (P) if the correct confidence interval is identified and constructed, but the interpretation is not in context or no interpretation is given.

OR

There are calculation errors with the appropriate confidence interval, but the interpretation follows correctly from the interval and is in context.

Incorrect (I) if the confidence interval and interpretation are not reasonable.

Section 3 [part (c) and part (d)] is scored as follows:

Essentially correct (E) if the student correctly (1) sets up and calculates the estimated relative risk, (2) sets up and calculates the confidence interval for the relative risk, and (3) interprets the confidence interval in context.

Partially correct (P) if the student provides only two correct components.

Incorrect (I) otherwise.

Section 4 [part (e)] is scored as follows:

Essentially correct (E) if the student (1) provides an advantage of using the confidence interval for the relative risk and (2) addresses both intervals.

Partially correct (P) if the first component is correct.

Incorrect (I) otherwise.

Each essentially correct (E) response counts as 1 point, and each partially correct (P) response counts as ½ point.

4  Complete Response
3  Substantial Response
2  Developing Response
1  Minimal Response

If a response is between two scores (for example, 2½ points), use a holistic approach to determine whether to score up or down, depending on the strength of the response and communication.
Directions: Show all your work. Indicate clearly the methods you use, because you will be graded on the correctness of your methods as well as on the accuracy and completeness of your results and explanations.

6. Two treatments, A and B, showed promise for treating a potentially fatal disease. A randomized experiment was conducted to determine whether there is a significant difference in the survival rate between patients who receive treatment A and those who receive treatment B. Of 154 patients who received treatment A, 38 survived for at least 15 years, whereas 16 of the 164 patients who received treatment B survived at least 15 years.

(a) Treatment A can be administered only as a pill, and treatment B can be administered only as an injection. Can this randomized experiment be performed as a double-blind experiment? Why or why not?

Yes, if one does not inform both patients and doctors about how the two treatments are applied, they will not know whether they gave/received Treatment A or Treatment B. Also, one can use a placebo for both treatments (i.e., giving sugar pills or injecting glucose).

(b) The conditions for inference have been met. Construct and interpret a 95 percent confidence interval for the difference between the proportion of the population who would survive at least 15 years if given treatment A and the proportion of the population who would survive at least 15 years if given treatment B.

\[
CI = \left(\hat{p}_A - \hat{p}_B\right) \pm z^* \sqrt{\frac{\hat{p}_A(1-\hat{p}_A)}{n_1} + \frac{\hat{p}_B(1-\hat{p}_B)}{n_2}}
\]

\[
= \left(\frac{38}{154} - \frac{16}{164}\right) \pm (1.960) \sqrt{\frac{\frac{38}{154}(1-\frac{38}{154})}{154} + \frac{\frac{16}{164}(1-\frac{16}{164})}{164}}
\]

\[
= 0.149 \pm (1.960)(0.042)
\]

\[
= \left(0.067, 0.231\right)
\]

We are 95% sure that the actual difference in the survival rate of two treatments are between 0.067 and 0.231.
In many of these types of studies, physicians are interested in the ratio of survival probabilities, $\frac{p_A}{p_B}$, where $p_A$ represents the true 15-year survival rate for all patients who receive treatment A and $p_B$ represents the true 15-year survival rate for all patients who receive treatment B. This ratio is usually referred to as the relative risk of the two treatments.

For example, a relative risk of 1 indicates the survival rates for patients receiving the two treatments are equal, whereas a relative risk of 1.5 indicates that the survival rate for patients receiving treatment A is 50 percent higher than the survival rate for patients receiving treatment B. An estimator of the relative risk is the ratio of estimated probabilities, $\frac{\hat{p}_A}{\hat{p}_B}$.

(c) Using the data from the randomized experiment described above, compute the estimate of the relative risk.

\[
\text{estimate of relative risk} = \frac{\hat{p}_A}{\hat{p}_B} = \frac{38/134}{16/134} = 2.53
\]
The sampling distribution of $\hat{p}_A / \hat{p}_B$ is skewed. However, when both sample sizes $n_A$ and $n_B$ are relatively large, the distribution of $\ln \left( \frac{\hat{p}_A}{\hat{p}_B} \right)$ — the natural logarithm of relative risk — is approximately normal with a mean of $\ln \left( \frac{1 - p_A}{1 - p_B} \right)$ and a standard deviation of $\sqrt{\frac{1 - p_A}{n_A p_A} + \frac{1 - p_B}{n_B p_B}}$, where $p_A$ and $p_B$ can be estimated by using $\hat{p}_A$ and $\hat{p}_B$.

When a 95 percent confidence interval for $\ln \left( \frac{p_A}{p_B} \right)$ is known, an approximate 95 percent confidence interval for $\frac{p_A}{p_B}$ — the relative risk of the two treatments — can be constructed by applying the inverse of the natural logarithm to the endpoints of the confidence interval for $\ln \left( \frac{p_A}{p_B} \right)$.

(d) The conditions for inference are met for the data in the experiment above, and a 95 percent confidence interval for $\ln \left( \frac{p_A}{p_B} \right)$ is $(0.3868, 1.4690)$. Construct and interpret a 95 percent confidence interval for the relative risk $\left( \frac{p_A}{p_B} \right)$ of the two treatments.

\[
\ln = \ln \left( \frac{p_A}{p_B} \right) = 0.3868
\]
\[
\Rightarrow \frac{p_A}{p_B} = e^{0.3868} \approx 1.47
\]
\[
\ln = \ln \left( \frac{p_A}{p_B} \right) = 1.47
\]
\[
\Rightarrow \frac{p_A}{p_B} = e^{1.4690} \approx 4.34
\]
\[
(C.I. = (1.47, 4.34))
\]

We are 95% confident that the actual relative risk of two treatments $\left( \frac{p_A}{p_B} \right)$ is between 1.47 and 4.34.
(e) What is an advantage of using the interval in part (d) over using the interval in part (b)?

Stating \( P_A \) as how many times greater than \( P_B \) (part d) rather than stating the difference \( P_A - P_B \) (part b) is more effective because the former represents small proportions better. For example, if \( P_A = 0.005 \) and \( P_B = 0.002 \), using part b results in 0.003 difference, whereas using part d results in relative risk of 2.5. Part b may lead to the bias that the difference in the two treatments is smaller than it actually is.

END OF EXAM

THE FOLLOWING INSTRUCTIONS APPLY TO THE COVERS OF THE SECTION II BOOKLET.

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STATISTICS
SECTION II
Part B
Question 6
Spend about 25 minutes on this part of the exam.
Percent of Section II score — 25

Directions: Show all your work. Indicate clearly the methods you use, because you will be graded on the correctness of your methods as well as on the accuracy and completeness of your results and explanations.

6. Two treatments, A and B, showed promise for treating a potentially fatal disease. A randomized experiment was conducted to determine whether there is a significant difference in the survival rate between patients who receive treatment A and those who receive treatment B. Of 154 patients who received treatment A, 38 survived for at least 15 years, whereas 16 of the 164 patients who received treatment B survived at least 15 years.

(a) Treatment A can be administered only as a pill, and treatment B can be administered only as an injection. Can this randomized experiment be performed as a double-blind experiment? Why or why not?

No. It will be obvious that people will understand that they get different treatments because of the difference between methods used to impose these two treatments. However, people who will collect the data about the number of patients who survived may be "blind"; they will not know what treatment the survived patient had received. But the researchers (who conduct experiment) will.

(b) The conditions for inference have been met. Construct and interpret a 95 percent confidence interval for the difference between the proportion of the population who would survive at least 15 years if given treatment A and the proportion of the population who would survive at least 15 years if given treatment B.

Conditions: 2 independent random samples

- Sample sizes are sufficiently large:
  - \( n_1 p_1 = 38 \), \( n_1 (1-p_1) = 154 - 38 \)
  - \( n_2 p_2 = 16 \), \( n_2 (1-p_2) = 164 - 16 \)
  - all are at least 5

So we may proceed. Confidence interval for differences in proportions:

\[
p_1 - p_2 \pm 2 \times \sqrt{\frac{p_1(1-p_1)}{n_1} + \frac{p_2(1-p_2)}{n_2}}
\]

\( \alpha/2 \) for 95% confidence level = 1.96

Confidence interval:

\[
0.2468 - 0.0976 + 1.96 \sqrt{\frac{38(1-0.38)}{154} + \frac{16(1-0.16)}{164}}
\]

\( = 0.1492 \pm 0.0822 \)

Based on this data, we are 95% confident that true difference in proportions of survived is between 6.7% and 23.14%.

GO ON TO THE NEXT PAGE.
In many of these types of studies, physicians are interested in the ratio of survival probabilities, $\frac{p_A}{p_B}$, where $p_A$ represents the true 15-year survival rate for all patients who receive treatment A and $p_B$ represents the true 15-year survival rate for all patients who receive treatment B. This ratio is usually referred to as the relative risk of the two treatments.

For example, a relative risk of 1 indicates the survival rates for patients receiving the two treatments are equal, whereas a relative risk of 1.5 indicates that the survival rate for patients receiving treatment A is 50 percent higher than the survival rate for patients receiving treatment B. An estimator of the relative risk is the ratio of estimated probabilities, $\hat{\frac{p_A}{p_B}}$.

(c) Using the data from the randomized experiment described above, compute the estimate of the relative risk:

$$\frac{\hat{p}_A}{\hat{p}_B} = \frac{p_A}{p_B} \pm \sqrt{\frac{\hat{\sigma}_A^2}{n_1} + \frac{\hat{\sigma}_B^2}{n_2}} = \frac{0.2468}{0.0976} \pm \sqrt{\frac{(\frac{p_A(1-p_A)}{n_1}) + (\frac{p_B(1-p_B)}{n_2})} = 2.5287 \pm 0.042}$$
The sampling distribution of $\frac{\hat{p}_A}{\hat{p}_B}$ is skewed. However, when both sample sizes $n_A$ and $n_B$ are relatively large, the distribution of $\ln \left( \frac{\hat{p}_A}{\hat{p}_B} \right)$ — the natural logarithm of relative risk — is approximately normal with a mean of $\ln \left( \frac{p_A}{p_B} \right)$ and a standard deviation of $\sqrt{\frac{1-p_A}{n_A p_A} + \frac{1-p_B}{n_B p_B}}$, where $p_A$ and $p_B$ can be estimated by using $\hat{p}_A$ and $\hat{p}_B$.

When a 95 percent confidence interval for $\ln \left( \frac{p_A}{p_B} \right)$ is known, an approximate 95 percent confidence interval for $\frac{p_A}{p_B}$ — the relative risk of the two treatments — can be constructed by applying the inverse of the natural logarithm to the endpoints of the confidence interval for $\ln \left( \frac{p_A}{p_B} \right)$.

(d) The conditions for inference are met for the data in the experiment above, and a 95 percent confidence interval for $\ln \left( \frac{p_A}{p_B} \right)$ is $(0.3868, 1.4690)$. Construct and interpret a 95 percent confidence interval for the relative risk, $\frac{p_A}{p_B}$, of the two treatments.

1. $\ln \left( \frac{p_A}{p_B} \right) = 0.3868$
   
   $\frac{p_A}{p_B} = e^{0.3868} = 1.47286$

2. $\ln \left( \frac{p_A}{p_B} \right) = 1.4690$
   
   $\frac{p_A}{p_B} = e^{1.4690} = 4.3449$

3. 95% confidence interval for $\frac{p_A}{p_B}$ is $(1.47286, 4.3449)$

   Based on these data, we can be 95% confident that the true relative risk $\frac{p_A}{p_B}$ of the two treatments is between 1.47286 and 4.3449.

**GO ON TO THE NEXT PAGE.**
(e) What is an advantage of using the interval in part (d) over using the interval in part (b)?

It takes into account not the difference between effectiveness, but estimate relative effectiveness by saying how many times treatment A is more effective than treatment B. It seems to be more vivid when the difference in treatments is claimed this way, and not by saying that one is more effective than the other by approximately 6% or 20%.

STOP

END OF EXAM

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6. Two treatments, A and B, showed promise for treating a potentially fatal disease. A randomized experiment was conducted to determine whether there is a significant difference in the survival rate between patients who receive treatment A and those who receive treatment B. Of 154 patients who received treatment A, 38 survived for at least 15 years, whereas 16 of the 164 patients who received treatment B survived at least 15 years.

(a) Treatment A can be administered only as a pill, and treatment B can be administered only as an injection. Can this randomized experiment be performed as a double-blind experiment? Why or why not?

This can **NOT** be administered as a double-blind experiment. Even if it is assumed that the patients know nothing about the treatments, the people administering the treatments would know which treatment is being administered to which patient because it is obvious from the way the treatment is administered.

(b) The conditions for inference have been met. Construct and interpret a 95 percent confidence interval for the difference between the proportion of the population who would survive at least 15 years if given treatment A and the proportion of the population who would survive at least 15 years if given treatment B.

A confidence interval for the difference between the two treatments is \([0.067, 0.231]\). This means that we are 95% confident that the true difference of proportions between the number of people who would survive 15 years is captured by this confidence interval. Also, because 0 does not fall into the confidence interval, at the 0.05 significance level, there is enough statistical evidence from this experiment to show that there is a difference between the effectiveness of the two treatments.
In many of these types of studies, physicians are interested in the ratio of survival probabilities, \( \frac{p_A}{p_B} \), where 

\( p_A \) represents the true 15-year survival rate for all patients who receive treatment A and \( p_B \) represents the true 15-year survival rate for all patients who receive treatment B. This ratio is usually referred to as the relative risk of the two treatments.

For example, a relative risk of 1 indicates the survival rates for patients receiving the two treatments are equal, whereas a relative risk of 1.5 indicates that the survival rate for patients receiving treatment A is 50 percent higher than the survival rate for patients receiving treatment B. An estimator of the relative risk is the ratio of estimated probabilities, \( \hat{p}_A/\hat{p}_B \).

(c) Using the data from the randomized experiment described above, compute the estimate of the relative risk.

Using the data, the estimated relative risk would be \( \frac{\hat{p}_A}{\hat{p}_B} = \frac{0.24675}{0.09756} = 2.53 \). The computed estimate of the relative risk between treatments A and B is 2.53, meaning the survival rate for patients receiving treatment A is 153% higher than patients receiving treatment B.
The sampling distribution of \( \frac{\hat{p}_A}{\hat{p}_B} \) is skewed. However, when both sample sizes \( n_A \) and \( n_B \) are relatively large, the distribution of \( \ln \left( \frac{\hat{p}_A}{\hat{p}_B} \right) \) — the natural logarithm of relative risk — is approximately normal with a mean of \( \ln \left( \frac{p_A}{p_B} \right) \) and a standard deviation of \( \sqrt{\frac{1-p_A}{n_A p_A} + \frac{1-p_B}{n_B p_B}} \), where \( p_A \) and \( p_B \) can be estimated by using \( \hat{p}_A \) and \( \hat{p}_B \).

When a 95 percent confidence interval for \( \ln \left( \frac{p_A}{p_B} \right) \) is known, an approximate 95 percent confidence interval for \( \frac{p_A}{p_B} \) — the relative risk of the two treatments — can be constructed by applying the inverse of the natural logarithm to the endpoints of the confidence interval for \( \ln \left( \frac{p_A}{p_B} \right) \).

(d) The conditions for inference are met for the data in the experiment above, and a 95 percent confidence interval for \( \ln \left( \frac{p_A}{p_B} \right) \) is (0.3868, 1.4690). Construct and interpret a 95 percent confidence interval for the relative risk, \( \frac{p_A}{p_B} \), of the two treatments.

A 95% confidence interval for the relative risk \( \frac{\hat{p}_A}{\hat{p}_B} \) is which is approximated by \( \frac{\hat{p}_A}{\hat{p}_B} = [1.47, 4.34] \). This means that we are confident that 95% of the true values of the relative risk are captured by this confidence interval. This confidence interval, because it does not include 0, also shows that there is evidence that A is more effective than treatment B.

GO ON TO THE NEXT PAGE.
(e) What is an advantage of using the interval in part (d) over using the interval in part (b) ?

The interval in part d, based on the relative risks of the two treatments, takes into account how many times higher the survival rate of patients using treatment A is than treatment B. Also, because the distribution is approximately normal for \( \ln(\frac{\theta_A}{\theta_B}) \), it is a safer assumption to make this confidence interval assumption.

STOP

END OF EXAM

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Question 6

Sample: 6A
Score: 4

In part (a) this response reflects understanding that a double-blind study requires that neither the patients nor the researchers will be able to detect which treatment is being administered and then suggests providing a placebo injection to those patients who receive treatment A and a placebo pill to those patients who receive treatment B. Thus section 1 [part (a)] was scored as essentially correct. In part (b) the confidence interval for the difference of two proportions is identified both by name (“2 prop. z-interval”) and formula. The mixed notation, " p_1 " and " p_2 " along with " p_A " and " p_B ", was considered a minor error. The interval is computed and interpreted correctly, although the student uses the phrase “95% sure” in place of the preferred words, 95 percent confident. Section 2 [part (b)] was therefore scored as essentially correct. Part (c) provides the correct value for the estimated relative risk, with work shown, and part (d) provides a correct confidence interval with interpretation. Thus section 3 was scored as essentially correct. Part (e) conveys the correct idea that when the proportions are small, reporting a confidence interval for how many times greater one proportion is than the other may better help people understand how the proportions differ than reporting a confidence interval for their difference, which can look insignificant. A nice example is included. Section 4 [part (e)] was scored as essentially correct. Because sections 1, 2, 3, and 4 were each scored essentially correct, this complete response received a score of 4.

Sample: 6B
Score: 3

Part (a) of this response does not include a method for making the study double-blind, but it does communicate that in a double-blind experiment the patients and the researchers must not know which treatments are assigned to which patients and that such would not be the case for the patient if one treatment were given by injection and the other by a pill. Thus section 1 was scored as partially correct. In part (b) it was not necessary to state or check the conditions for constructing a confidence interval for the difference of two proportions. The formula for the interval, with correct computations and a correct interpretation, are provided, so section 2 was scored as essentially correct. Part (c) provides the correct value for the estimated relative risk, with work shown, and part (d) provides a correct confidence interval with interpretation. Thus section 3 was scored as essentially correct. Part (e) conveys the correct idea that when the proportions are small, reporting a confidence interval for how many times more effective one treatment is than another seems more “vivid” in contrast with the interval computed in part (b), where the difference in the proportions appears small. Section 4 was therefore scored as essentially correct. Because sections 2 and 4 were essentially correct, and sections 1 and 3 were partially correct, this substantial response received a score of 3.

Sample: 6C
Score: 2

Part (a) of this response does not include a method for making the study double-blind, but it does communicate that in a double-blind experiment the patients and the medical practitioners must not know which treatment is being administered and that such would not be the case, at least for the practitioners, if one treatment is given by injection and the other by a pill. Thus, section 1 was scored as partially correct. In part (b) the confidence interval is correct and the interpretation is excellent, but neither the name of the test nor the formula is included. Thus section 2 was scored as partially correct. The relative risk in part (c) and the confidence interval in part (d) are computed correctly. However, the interpretation of the confidence interval in part (d) is incorrect. The student refers to “the true values of the relative risk” when in fact there is only one true value of this risk. Furthermore, when using this confidence interval, one
would be interested in whether it contained 1 (rather than 0). Thus section 3 was scored as partially correct. The first sentence of part (e) makes a correct statement about relative risk, but this is not an advantage of using a confidence interval for relative risk rather than a confidence interval for the difference of the two proportions. The first part of the second sentence is true as well, but the sampling distribution for the difference of two proportions also is approximately normal, so normality is not an advantage for the interval of part (d). Section 4 was therefore scored as incorrect. Because sections 1, 2, and 3 were each partially correct and section 4 was incorrect, this developing response received a score of 2.