Chapter 7

Power Analysis of Proportions

Introduction

This chapter introduces power analysis and sample size calculation for proportions. When the response is binary, the results for each group may be summarized as proportions. Usually, hypothesis tests are conducted to compare two proportions.

There are many issues that must be considered when designing experiments that use proportions. For example, will the proportions be analyzed directly, or will they be converted into differences, ratios, or odds ratios? Which test statistic will be used? Will the design use independent groups or will subjects be measured twice? Will the study have an active control?

The various answers to these and other questions result in a large number of situations. This chapter will introduce you to the issues that are common to all the tests of proportions.

Designs

There are several experimental designs for comparing two proportions. You can you a one-sample design to compare a sample proportion to a specific value. You can compare proportions from two independent samples using independent, stratified, cluster-randomized, or group-sequential designs. You can compare two correlated proportions. And finally, you can compare several categories in a contingency table.

Comparing Proportions

Two proportions may be compared by considering their difference, ratio, or odds ratio. Each of these cases may lead to different test statistics with different powers and sample size requirements.

Assume that p_1 is the response proportion of the experimental group and p_2 is the response proportion of the control (standard or reference) group. Mathematically, these alternative parameterizations are

Parameter	Computation
Difference	$\delta = p_1 - p_2$
Ratio	$\phi = p_1 / p_2$
Odds Ratio	$\psi = \frac{p_1/(1-p_1)}{p_2/(1-p_2)}$

Once you know p_1 and p_2, you can calculate any of these values, and you can easily change from once parameterization to another. Thus, the choice of which parameter you use may seem arbitrary. However, since different parameterizations lead to different test statistics, the choice can lead to a different power and sample size. These parameterizations will be discussed next.

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Difference

The difference $\delta = p_1 - p_2$ is perhaps the most common method of comparing two proportions. This parameter is easy to interpret and communicate. It gives the absolute impact of the treatment. However, there are subtle difficulties that can arise with its use.

One difficulty occurs when the event of interest is rare. If a difference of 0.001 is reported for an event with a baseline probability of 0.40, we would dismiss this as being trivial. That is, there is usually little interest in a treatment that decreases the probability from 0.400 to 0.399. However, if the baseline probability of a disease is 0.002, a 0.001 decrease in the disease probability would represent a 50% reduction. Thus, the interpretation of the difference depends on the baseline probability of the event.

When planning studies, the value of p_2 is usually known and the power is calculated at various values of δ. The value of p_1 is then calculated using $p_1 = p_2 + \delta$. Because of the requirement that $0 < p_1 < 1$, the values of δ are restricted to the interval $-p_2 < \delta < 1 - p_2$, not $-1 < \delta < 1$ as you might expect. Likewise, the values of p_2 are restricted to $0 < p_2 < 1 - \delta$ if $\delta > 0$ and $-\delta < p_2 < 1$ if $\delta < 0$.

Because typical values of δ are usually between -0.2 and 0.2, the allowable values of p_2 are restricted to be between 0.2 and 0.8. When the values of p_2 are outside this range, it may be more convenient to use the odds ratio.

Ratio

The (risk) ratio, $\phi = p_1/p_2$, gives the relative change in the probability of the outcome under each of the hypothesized values. This parameter is direct and easy to interpret. To compare the ratio with the difference, examine the case where $p_1 = 0.1437$ and $p_2 = 0.0793$. One should consider which number is more enlightening, $\delta = -0.0644$, or $\phi = 55.18\%$. In many cases, the relative change (the ratio) is easier to interpret that the absolute change (the difference).

When planning studies, the value of p_2 is usually known and the power is calculated at various values of ϕ. The value of p_1 is then calculated using $p_1 = p_2 \times \phi$. Because of the requirement that $0 < p_1 < 1$, the values of ϕ are restricted to the interval $0 < \phi < 1/p_2$, not $0 < \phi < \infty$ as you might expect. Likewise, the values of p_2 are restricted to $0 < p_2 < 1/\phi$ if $\phi > 1$ and $0 < p_2 < 1$ if $\phi < 1$.

Because typical values of ϕ are usually between 0.5 and 1.5, the values of p_2 are restricted to be between 0 and 0.667. When the values of p_2 are outside this range, it may be more convenient to use the odds ratio.

Odds Ratio

Chances are usually communicated as long-term proportions or probabilities. In betting, chances are often given as odds. For example, the odds of a horse winning a race might be set at 10-to-1 or 3-to-2. Odds can easily be translated into probabilities, and vice versa. An odds of 3-to-2 means that the event is expected to occur three out of five times. That is, an odds of 3-to-2 (1.5) translates to a probability of winning of 0.60.

The odds of an event are calculated by dividing the event risk by the non-event risk. Thus, the odds are

$$Odds_1 = \frac{p_1}{1 - p_1} \quad \text{and} \quad Odds_2 = \frac{p_2}{1 - p_2}$$

For example, if p is 0.6, the odds are 0.6/0.4 = 1.5. Rather than represent the odds as a decimal amount, it is re-scaled into whole numbers. Thus, instead of presenting the odds as 1.5-to-1, they present as 3-to-2.

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Two odds could be compared by considering their difference, but it is more convenient in many situations to form their ratio. Thus, another way to compare proportions is to compute the ratio of their odds. The odds ratio is

$$\psi = \frac{Odds_1}{Odds_2}$$

$$= \frac{\frac{p_1}{1-p_1}}{\frac{p_2}{1-p_2}}$$

Unlike the difference and the ratio, the odds ratio is not restricted by the value of p_2. The range of possible values of the odds ratio is $-\infty < \psi < \infty$. Because of the freedom in specifying the parameters, the odds ratio is a popular parameterization, even though it is not as easy to interpret as the difference and the ratio.

Specifying the Proportions – Important!

It is important to understand the interpretation of p_1 and p_2 within **PASS**. Suppose p_1 represents the proportion in the treatment group and p_2 represents the proportion in the control group. In most hypothesis tests, these values are equal under the null hypothesis and different under the alternative hypothesis. Thus, under the null hypothesis, all that is needed is the value of p_1 or p_2, but not both. Under the alternative hypothesis, both values are necessary. So, when the input screen asks for p_2 and the difference, these values should be interpreted as follows. The value of p_2 is actually the value of both p_1 and p_2 under H_0. Under H_1, the value of p_1 is calculated from p_2 and δ using the formula $p_1 = p_2 + \delta$.

Also, it is important to understand what we mean by "under H_1" in the above discussion. Notice that H_1 does not specify the exact value of p_1. Instead, it specifies a range of values. For example, H_1 might be $p_1 > p_2$ or $p_1 - p_2 > \delta$. However, even though H_1 gives a range of values for p_1, the power is computed at a specific value of p_1.

Selecting an appropriate value for p_1 must be done very carefully. We recommend the following approach. Select a value of p_1 that represents the change from p_2 that you want the experiment to detect. When you calculate a sample size, it is interpreted as the sample size necessary to detect a difference of at least $p_1 - p_2$ when the significance level is α and the power is 1 – β.

The important point is that p_1 is not the value you anticipate obtaining from an experiment. Instead, it is that value of p_1 at which you want to compute the power. This is a very important distinction! This is why, when investigating the power after an experiment is run, we recommend that you do not simply plug in the values of p_1 and p_2 from that experiment. Rather, you use values that represent the size of the difference that you want to detect.

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