Chapter 198

# Tests for One Proportion using Effect Size

## Introduction

This procedure provides sample size and power calculations for one- or two-sided hypothesis tests of the difference between a proportion and a given value (between 0 and 1) using the effect size. The details of this procedure are given in Cohen (1988). The design corresponding to this test procedure is sometimes referred to as a *one-arm* design versus a historical control. In this design, two proportions are compared by considering their difference. This difference is formed between transformed values of the proportions, formed to create variables that are more normally distributed than the raw proportions and that have a variance not related to the values of the proportions.

## Test Procedure

If we assume that $P_1$ and $P_0$ represent the two proportions. $P1$ represents the population being studied. $P0$ is the historical control proportion. The effect size is represented by the difference $h$ formed as follows

$$h = \varphi_1 - \varphi_0$$

where

$$\varphi_i = 2\,\text{arcsine}\left(\sqrt{P_i}\right)$$

This is referred to as the arcsine, the arcsine root, or the angular transformation.

The null hypothesis is $H_0$: h = 0 and the alternative hypothesis depends on the number of "sides" of the test:

| | | |
|---|---|---|
| Two-Sided: | $H_1: h \neq 0$ | or $H_1: \varphi_1 - \varphi_0 \neq 0$ |
| Upper One-Sided: | $H_1: h > 0$ | or $H_1: \varphi_1 - \varphi_0 > 0$ |
| Lower One-Sided: | $H_1: h < 0$ | or $H_1: \varphi_1 - \varphi_0 < 0$ |

A suitable Type I error probability ($\alpha$) is chosen for the test, the data is collected, and a $z$-statistic is generated using the formula

$$z = \frac{\hat{\varphi}_1 - \varphi_0}{\sqrt{\frac{1}{N}}}$$

This *z*-statistic follows a standard normal distribution. The null hypothesis is rejected in favor of the alternative if,

$$\text{for} \quad H_1: h \neq 0, \quad z < z_{\alpha/2} \quad \text{or} \quad z > z_{1-\alpha/2}$$

$$\text{for} \quad H_1: h > 0, \quad z > z_{1-\alpha}$$

$$\text{for} \quad H_1: h < 0, \quad z < z_{\alpha}$$

Comparing the z-statistic to the cut-off *z*-value is equivalent to comparing the *p*-value to $\alpha$.

# Power Calculation

The power of a one-sided test is calculated using the formulation of Cohen (1988):

$$z_{1-\beta} = h\sqrt{N} - z_{1-\alpha}$$

# The Effect Size

As stated above, the effect size *h* is given by $h = \varphi_1 - \varphi_0$. Cohen (1988) proposed the following interpretation of the *h* values. An *h* near 0.2 is a *small* effect, an *h* near 0.5 is a *medium* effect, and an *h* near 0.8 is a *large* effect. These values for small, medium, and large effects are popular in the social sciences.

Cohen (1988) remarks that the value of h does not match directly with the value of $P_1 - P_0$, so care must be taken when using it. For example, all of the following pairs of values of $P_1$ and $P_0$ result in an *h* of about 0.30, even though the actual differences $P_1 - P_0$ are quite different.

| $P_1$ | $P_0$ | $P_1 - P_0$ | h |
|---|---|---|---|
| 0.21 | 0.10 | 0.11 | 0.3 |
| 0.39 | 0.25 | 0.14 | 0.3 |
| 0.55 | 0.40 | 0.15 | 0.3 |
| 0.65 | 0.50 | 0.15 | 0.3 |
| 0.78 | 0.60 | 0.18 | 0.3 |
| 0.87 | 0.75 | 0.13 | 0.3 |
| 0.97 | 0.90 | 0.07 | 0.3 |

# Example 1 – Finding the Sample Size

Researchers wish to compare a new type of local anesthesia to the commonly used type using a one-sample design. Subjects in pain will be studied. The treatment will be administered, and the subject's evaluation of pain intensity will be measured on a binary scale (acceptable, unacceptable).

The researchers would like to determine the sample sizes required to detect a small, medium, and large effect size with a two-sided z test when the power is 80% or 90% and the significance level is 0.05.

## Setup

If the procedure window is not already open, use the PASS Home window to open it. The parameters for this example are listed below and are stored in the **Example 1** settings file. To load these settings to the procedure window, click **Open Example Settings File** in the Help Center or File menu.

| Design Tab | |
|---|---|
| Solve For | **Sample Size** |
| Alternative Hypothesis | **Two-Sided** |
| Power | **0.80 0.90** |
| Alpha | **0.05** |
| h | **0.2 0.5 0.8** |

## Output

Click the Calculate button to perform the calculations and generate the following output.

### Numeric Reports

**Numeric Results**

| | |
|---|---|
| Solve For: | Sample Size |
| Test Type: | Z-Test |
| Alternative Hypothesis: | H1: h ≠ 0 |

| Power | | Sample Size | Effect Size | |
|---|---|---|---|---|
| Target | Actual | N | h | Alpha |
| 0.8 | 0.8016 | 197 | 0.2 | 0.05 |
| 0.9 | 0.9003 | 263 | 0.2 | 0.05 |
| 0.8 | 0.8074 | 32 | 0.5 | 0.05 |
| 0.9 | 0.9064 | 43 | 0.5 | 0.05 |
| 0.8 | 0.8224 | 13 | 0.8 | 0.05 |
| 0.9 | 0.9096 | 17 | 0.8 | 0.05 |

| | |
|---|---|
| Target Power | The desired power. It may not be achieved because of integer N. |
| Actual Power | The achieved power. Because N is an integer, this value is often (slightly) larger than the target power. |
| N | The number of items sampled from the population. |
| h | Effect Size. Cohen recommended Low = 0.2, Medium = 0.5, and High = 0.8. h = φ1 - φ0, where $\varphi_i = 2 \times \text{ArcSine}(\sqrt{P_i})$. |
| Alpha | The probability of rejecting a true null hypothesis. |

### Summary Statements

A single-group (one-arm) design will be used to test whether the proportion effect size ($h = \varphi 1 - \varphi 0$, where $\varphi_i = 2 \times \text{ArcSine}(\sqrt{P_i})$) is different from 0 ($H0: h = 0$ versus $H1: h \neq 0$). The comparison will be made using a two-sided, one-sample Z-test, with a Type I error rate ($\alpha$) of 0.05. To detect an effect size of 0.2 with 80% power, 197 subjects will be needed.

### Dropout-Inflated Sample Size

| Dropout Rate | Sample Size N | Dropout-Inflated Enrollment Sample Size N' | Expected Number of Dropouts D |
|---|---|---|---|
| 20% | 197 | 247 | 50 |
| 20% | 263 | 329 | 66 |
| 20% | 32 | 40 | 8 |
| 20% | 43 | 54 | 11 |
| 20% | 13 | 17 | 4 |
| 20% | 17 | 22 | 5 |

| | |
|---|---|
| Dropout Rate | The percentage of subjects (or items) that are expected to be lost at random during the course of the study and for whom no response data will be collected (i.e., will be treated as "missing"). Abbreviated as DR. |
| N | The evaluable sample size at which power is computed. If N subjects are evaluated out of the N' subjects that are enrolled in the study, the design will achieve the stated power. |
| N' | The total number of subjects that should be enrolled in the study in order to obtain N evaluable subjects, based on the assumed dropout rate. After solving for N, N' is calculated by inflating N using the formula N' = N / (1 - DR), with N' always rounded up. (See Julious, S.A. (2010) pages 52-53, or Chow, S.C., Shao, J., Wang, H., and Lokhnygina, Y. (2018) pages 32-33.) |
| D | The expected number of dropouts. D = N' - N. |

### Dropout Summary Statements

Anticipating a 20% dropout rate, 247 subjects should be enrolled to obtain a final sample size of 197 subjects.

### References

Cohen, Jacob. 1988. Statistical Power Analysis for the Behavioral Sciences. Lawrence Erlbaum Associates. Hillsdale, New Jersey

Julious, S. A. 2010. Sample Sizes for Clinical Trials. Chapman & Hall/CRC. Boca Raton, FL.

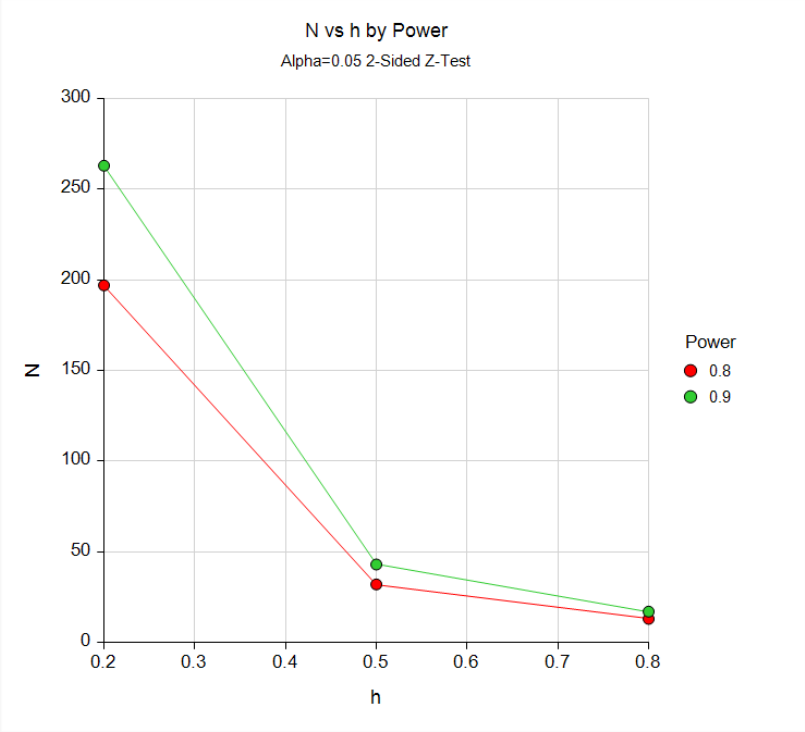
Machin, D., Campbell, M., Tan, B. T., Tan, S. H. 2009. Sample Size Tables for Clinical Studies, 3rd Edition. Wiley-Blackwell.

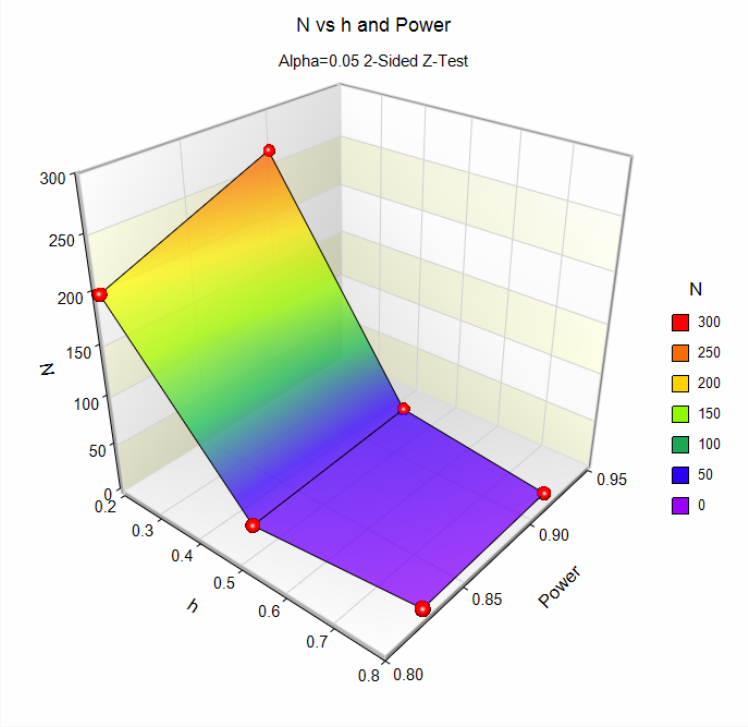
Ryan, Thomas P. 2013. Sample Size Determination and Power. John Wiley & Sons. New Jersey.

These reports show the values of each of the parameters, one scenario per row.

## Plots Section

**Plots**




These plots show the relationship between effect size, power, and sample size.

# Example 2 – Validation using Cohen (1988)

Cohen (1988) gives an example on page 208 of a two-sided test in which alpha = 0.05, h = 0.2, and power =0.95. He finds the sample size to be 325.

## Setup

If the procedure window is not already open, use the PASS Home window to open it. The parameters for this example are listed below and are stored in the **Example 2** settings file. To load these settings to the procedure window, click **Open Example Settings File** in the Help Center or File menu.

| Design Tab | |
|---|---|
| Solve For | **Sample Size** |
| Alternative Hypothesis | **Two-Sided** |
| Power | **0.95** |
| Alpha | **0.05** |
| h | **0.2** |

## Output

Click the Calculate button to perform the calculations and generate the following output.

**Numeric Results**

| | |
|---|---|
| Solve For: | Sample Size |
| Test Type: | Z-Test |
| Alternative Hypothesis: | H1: h ≠ 0 |

| Power | | Sample Size | Effect Size | |
|---|---|---|---|---|
| Target | Actual | N | h | Alpha |
| 0.95 | 0.9501 | 325 | 0.2 | 0.05 |

**PASS** also calculated the sample size to be 325 which validates the procedure.