**Chapter 253**

# Tests for Two Ordered Categorical Variables

## Introduction

This module computes power and sample size for tests of ordered categorical data such as Likert scale data. Assuming proportional odds, such data can be analyzed directly with a z-test, or using logistic regression or the Mann-Whitney test. The power and sample size formulae presented here are consistent with any of these analysis methods. The results used here were presented in a paper by Whitehead (1993). They are also mentioned in the book by Julious (2010) and Machin et al (1997).

Ordered categorical data often results from surveys such as a quality of life (QoL) survey in which responses are categories such as *very good, good, moderate, poor*. When there are only two categories, an analysis using two proportions should be used. When there are more than two responses, and those responses can be ordered, the techniques described in this chapter can be used.

## Technical Details

Suppose a variable has $K$ possible responses $C_1,...,C_K$. Further suppose that these categories can be ordered so that $C_i$ is more desirable than $C_j$ if $i < j$. Hence $C_1$ is the best outcome and $C_K$ is the worst. This procedure compares the results from two groups which will be called control (C) and experimental (E). The number of respondents falling within the $i^{th}$ category of the control group is labeled $N_{i1}$. The total number in the control group is $N_1$ and in the experimental group is $N_2$. The total sample size of the study is $N = N_1 + N_2$.

Let $p_{iE}$ denote the probability that an individual in the experimental group gives response $C_i$, and let $Q_{iE}$ be the probability of an outcome of $C_i$ or better. Thus $Q_{iE} = \sum_{j=1}^{i} p_{jE}$. Define $p_{iC}$ and $Q_{iC}$ similarly for the control group. Define the log-odds ratio for a particular category as

$$\theta_i = \log\left\{\frac{\dfrac{Q_{iE}}{1-Q_{iE}}}{\dfrac{Q_{iC}}{1-Q_{iC}}}\right\} \quad i = 1,\ldots,K-1.$$

This measures the advantage of the experimental group over the control group. A positive $\theta_i$ indicates that the experimental treatment is better than the control treatment.

## Proportional Odds

The proportional odds model assumes that all of these log-odds ratios are equal to a common value $\theta$. That is, the *proportional odds* assumption is that $\theta_1 = \cdots = \theta_{K-1} = \theta$. Thus the whole pattern of response differences can be summarized a single parameter.

The formulae to follow use the fact that efficient score $Y$ is asymptotically normally distributed when $\theta$ is small and $n$ is large. The test statistic and power formulae are as follows.

$$Y = \frac{1}{N+1}\sum_{i=1}^{k} N_{i1}\left(L_{iC} - U_{iC}\right)$$

$$Z = \frac{Y - \mu_Y}{\sigma_Y}$$

$$L_{iC} = \sum_{j=1}^{i-1} N_{j1}, \quad i = 2,\ldots,K$$

$$U_{iC} = \sum_{j=i+1}^{K} N_{j1}, \quad i = 1,\ldots,K-1$$

$$L_{1C} = U_{KC} = 0$$

$$\mu_Y = \theta V$$

$$\sigma_Y^2 = V$$

$$V = \frac{N_1 N_2 N}{3(N+1)^2}\left\{1 - \sum_{i=1}^{K}\left(\frac{N_{i1} + N_{i2}}{N}\right)^3\right\}$$

$$\approx \frac{N_1 N_2 N}{3(N+1)^2}\left\{1 - \sum_{i=1}^{K}\left(\frac{p_{iE} + p_{iC}}{2}\right)^3\right\}$$

The null hypothesis $H_0 : \theta = 0$ (the two treatments are equivalent) can be tested against the alternative $H_a : \theta \neq 0$ by computing $Z$ and rejecting if $Z$ is greater than $z_{\alpha/2}$. That is,

$$P(Z > z_\alpha \mid \theta = 0) = \alpha/2$$

The power is the probability of rejecting a false null hypothesis, thus the power for a specified value $\theta_R$ is

$$Power = P(Z > z_{\alpha/2} \mid \theta = \theta_R)$$
$$= 1 - \Phi\left(z_{\alpha/2} - \theta_R \sqrt{V}\right)$$

If a one-sided test is needed, replace $\alpha/2$ with $\alpha$.

# Procedure Options

This section describes the options that are specific to this procedure. These are located on the Design tab. For more information about the options of other tabs, go to the Procedure Window chapter.

## Design Tab

The Design tab contains most of the parameters and options that you will be concerned with.

### Solve For

#### Solve For

This option specifies the parameter to be solved for from the other parameters. The parameters that may be selected are $\theta$, *Sample Size (N1)*, *Sample Size (N2)*, or *Power*.

### Test

#### Null Hypothesis

Indicate whether the hypothesis is one-sided or two-sided. If *two-sided* is selected, alpha is automatically divided by 2, so you do not need to half alpha.

### Power and Alpha

#### Power

This option specifies one or more values for power. Power is the probability of rejecting a false null hypothesis, and is equal to one minus beta. Beta is the probability of a type-II error, which occurs when a false null hypothesis is not rejected. In this procedure, a type-II error occurs when you fail to reject the null hypothesis of $\theta = 0$ when, in fact, $\theta > 0$.

Values must be between zero and one. Historically, the value of 0.80 (Beta = 0.20) was used for power. Now, 0.90 (Beta = 0.10) is also commonly used.

A single value may be entered here or a range of values such as *0.8 to 0.95 by 0.05* may be entered.

#### Alpha

This option specifies one or more values for the probability of a type-I error. A type-I error occurs when a true null hypothesis is rejected. In this procedure, a type-I error occurs when you reject the null hypothesis that $\theta = 0$ when in fact it is.

Values of alpha must be between zero and one. Historically, the value of 0.05 has been used for alpha. This means that about one test in twenty will falsely reject the null hypothesis. You should pick a value for alpha that represents the risk of a type-I error you are willing to take in your experimental situation.

You may enter a range of values such as *0.01 0.05 0.10* or *0.01 to 0.10 by 0.01*.

Note that when you are analyzing a two-sided test, you should enter alpha, not alpha/2.

## Sample Size (When Solving for Sample Size)

### Group Allocation

Select the option that describes the constraints on *N1* or *N2* or both.

The options are

- **Equal (N1 = N2)**

  This selection is used when you wish to have equal sample sizes in each group. Since you are solving for both sample sizes at once, no additional sample size parameters need to be entered.

- **Enter N1, solve for N2**

  Select this option when you wish to fix *N1* at some value (or values), and then solve only for *N2*. Please note that for some values of *N1*, there may not be a value of *N2* that is large enough to obtain the desired power.

- **Enter N2, solve for N1**

  Select this option when you wish to fix *N2* at some value (or values), and then solve only for *N1*. Please note that for some values of *N2*, there may not be a value of *N1* that is large enough to obtain the desired power.

- **Enter R = N2/N1, solve for N1 and N2**

  For this choice, you set a value for the ratio of *N2* to *N1*, and then PASS determines the needed *N1* and *N2*, with this ratio, to obtain the desired power. An equivalent representation of the ratio, *R*, is

  $$N2 = R * N1.$$

- **Enter percentage in Group 1, solve for N1 and N2**

  For this choice, you set a value for the percentage of the total sample size that is in Group 1, and then PASS determines the needed *N1* and *N2* with this percentage to obtain the desired power.

### N1 (Sample Size, Group 1)

*This option is displayed if Group Allocation = "Enter N1, solve for N2"*

*N1* is the number of items or individuals sampled from the Group 1 population.

*N1* must be $\geq 2$. You can enter a single value or a series of values.

### N2 (Sample Size, Group 2)

*This option is displayed if Group Allocation = "Enter N2, solve for N1"*

*N2* is the number of items or individuals sampled from the Group 2 population.

*N2* must be $\geq 2$. You can enter a single value or a series of values.

### R (Group Sample Size Ratio)

*This option is displayed only if Group Allocation = "Enter R = N2/N1, solve for N1 and N2."*

*R* is the ratio of *N2* to *N1*. That is,

$$R = N2 / N1.$$

Use this value to fix the ratio of *N2* to *N1* while solving for *N1* and *N2*. Only sample size combinations with this ratio are considered.

*N2* is related to *N1* by the formula:

$$N2 = [R \times N1],$$

where the value *[Y]* is the next integer $\geq Y$.

For example, setting *R* = 2.0 results in a Group 2 sample size that is double the sample size in Group 1 (e.g., *N1* = 10 and *N2* = 20, or *N1* = 50 and *N2* = 100).

*R* must be greater than 0. If *R* < 1, then *N2* will be less than *N1*; if *R* > 1, then *N2* will be greater than *N1*. You can enter a single or a series of values.

### Percent in Group 1

*This option is displayed only if Group Allocation = "Enter percentage in Group 1, solve for N1 and N2."*

Use this value to fix the percentage of the total sample size allocated to Group 1 while solving for *N1* and *N2*. Only sample size combinations with this Group 1 percentage are considered. Small variations from the specified percentage may occur due to the discrete nature of sample sizes.

The Percent in Group 1 must be greater than 0 and less than 100. You can enter a single or a series of values.

## Sample Size (When <u>Not</u> Solving for Sample Size)

### Group Allocation

Select the option that describes how individuals in the study will be allocated to Group 1 and to Group 2.

The options are

- **Equal (N1 = N2)**

  This selection is used when you wish to have equal sample sizes in each group. A single per group sample size will be entered.

- **Enter N1 and N2 individually**

  This choice permits you to enter different values for *N1* and *N2*.

- **Enter N1 and R, where N2 = R * N1**

  Choose this option to specify a value (or values) for *N1*, and obtain *N2* as a ratio (multiple) of *N1*.

- **Enter total sample size and percentage in Group 1**

  Choose this option to specify a value (or values) for the total sample size (*N*), obtain *N1* as a percentage of *N*, and then *N2* as *N - N1*.

**Tests for Two Ordered Categorical Variables**

## Sample Size Per Group

*This option is displayed only if Group Allocation = "Equal (N1 = N2)."*

The Sample Size Per Group is the number of items or individuals sampled from each of the Group 1 and Group 2 populations. Since the sample sizes are the same in each group, this value is the value for *N1*, and also the value for *N2*.

The Sample Size Per Group must be ≥ 2. You can enter a single value or a series of values.

## N1 (Sample Size, Group 1)

*This option is displayed if Group Allocation = "Enter N1 and N2 individually" or "Enter N1 and R, where N2 = R * N1."*

*N1* is the number of items or individuals sampled from the Group 1 population.

*N1* must be ≥ 2. You can enter a single value or a series of values.

## N2 (Sample Size, Group 2)

*This option is displayed only if Group Allocation = "Enter N1 and N2 individually."*

*N2* is the number of items or individuals sampled from the Group 2 population.

*N2* must be ≥ 2. You can enter a single value or a series of values.

## R (Group Sample Size Ratio)

*This option is displayed only if Group Allocation = "Enter N1 and R, where N2 = R * N1."*

*R* is the ratio of *N2* to *N1*. That is,

$$R = N2/N1$$

Use this value to obtain *N2* as a multiple (or proportion) of *N1*.

*N2* is calculated from *N1* using the formula:

$$N2=[R \times N1],$$

where the value *[Y]* is the next integer ≥ *Y*.

For example, setting *R* = 2.0 results in a Group 2 sample size that is double the sample size in Group 1.

*R* must be greater than 0. If *R* < 1, then *N2* will be less than N1; if *R* > 1, then *N2* will be greater than *N1*. You can enter a single value or a series of values.

## Total Sample Size (N)

*This option is displayed only if Group Allocation = "Enter total sample size and percentage in Group 1."*

This is the total sample size, or the sum of the two group sample sizes. This value, along with the percentage of the total sample size in Group 1, implicitly defines *N1* and *N2*.

The total sample size must be greater than one, but practically, must be greater than 3, since each group sample size needs to be at least 2.

You can enter a single value or a series of values.

## Percent in Group 1

*This option is displayed only if Group Allocation = "Enter total sample size and percentage in Group 1."*

This value fixes the percentage of the total sample size allocated to Group 1. Small variations from the specified percentage may occur due to the discrete nature of sample sizes.

The Percent in Group 1 must be greater than 0 and less than 100. You can enter a single value or a series of values.

## Effect Size

### Control Group Category Proportions

This option contains the $p_{iC}$, the proportions in each category of the control group. The number of categories is implied by the number of items in the list.

For example, an entry of "20 30 40 10" implies that there are four categories and that 20% are category 1, 30% are category 2, 40% are category 3, and 10% are category 4.

The first category is the assumed to be the best outcome and the last category is the worst. Also, the categories are assumed to be ordered so that each is no better than the previous category. For example, you might have categories *Very Good, Good, Neutral, Poor, Very Poor*.

Note that the values entered here should to one. If they do not, they are scaled so that they do. For example, the values "20 30 40 10" (which sum to 100) are scaled to "0.2 0.3 0.4 0.1" (which sum to one).

You can enter any list of positive, non-zero numbers. For example, if you wanted to indicate that you have five, equally-likely categories, you could enter "1 1 1 1 1."

### θ (Log Odds Ratio of Treatment Group)

Enter one or more values of $\theta$, the logarithm of the odds. This value represents the effect size to be detected by the study. Note that the log odds ratio is assumed to be constant for all categories.

**Examples**

0.25 0.5 0.75 1.0

0.8 to 1.4 by 0.2

# Example 1 – Finding the Power

Suppose a clinical trial is planned to compare the response, made by a doctor, to certain treatment. The subjects are divided into two groups: those that will receive the current treatment and those that will receive an experimental treatment. Three months after the administration of the treatment, the doctor rates the response as *very good, good, moderate,* or *poor.* Historically, the responses have been about 20% *very good*, 50% *good*, 20% *moderate*, and 10% *poor.*

The researchers want to consider a range of possible value of $\theta$ from 0.5 to 2.0. They want to look at the power achieved by sample sizes from 30 to 150 per group. They want to set alpha to 0.05 and analyze the results with a two-sided test.

## Setup

This section presents the values of each of the parameters needed to run this example. First, from the PASS Home window, load the **Tests for Two Ordered Categorical Variables** procedure window by expanding **Proportions**, then clicking on **Ordered Categorical**, and then clicking on **Tests for Two Ordered Categorical Variables**. You may then make the appropriate entries as listed below, or open **Example 1** by going to the **File** menu and choosing **Open Example Template**.

| Option | Value |
|---|---|
| **Design Tab** | |
| Solve For ................................................. | **Power** |
| Null Hypothesis....................................... | **Two-Sided** |
| Alpha....................................................... | **0.05** |
| Group Allocation ..................................... | **Equal (N1 = N2)** |
| Sample Size Per Group .......................... | **30 to 150 by 10** |
| Control Group Category Proportions ...... | **20 50 20 10** |
| θ (Log Odds Ratio of Treatment Group). | **0.5 to 2.0 by 0.5** |

## Annotated Output

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

### Numeric Results

**Numeric Results for Two-Sample, Ordered-Categorical Test**
Null Hypothesis:  $\theta = 0$     Alternative Hypothesis: $\theta \neq 0$

| Power | N1 | N2 | N | Trt<br>Log Odds<br>Ratio<br>θ | Control<br>Categ. 1<br>Prop.<br>PC(1) | Trt<br>Categ. 1<br>Prop.<br>PE(1) | Alpha |
|---|---|---|---|---|---|---|---|
| 0.1726 | 30 | 30 | 60 | 0.5000 | 0.2000 | 0.2919 | 0.050 |
| 0.5310 | 30 | 30 | 60 | 1.0000 | 0.2000 | 0.4046 | 0.050 |
| 0.8653 | 30 | 30 | 60 | 1.5000 | 0.2000 | 0.5284 | 0.050 |
| 0.9828 | 30 | 30 | 60 | 2.0000 | 0.2000 | 0.6488 | 0.050 |
| 0.2172 | 40 | 40 | 80 | 0.5000 | 0.2000 | 0.2919 | 0.050 |
| 0.6564 | 40 | 40 | 80 | 1.0000 | 0.2000 | 0.4046 | 0.050 |
| 0.9444 | 40 | 40 | 80 | 1.5000 | 0.2000 | 0.5284 | 0.050 |
| 0.9972 | 40 | 40 | 80 | 2.0000 | 0.2000 | 0.6488 | 0.050 |
| 0.2613 | 50 | 50 | 100 | 0.5000 | 0.2000 | 0.2919 | 0.050 |
| 0.7543 | 50 | 50 | 100 | 1.0000 | 0.2000 | 0.4046 | 0.050 |
| . | . | . | . | . | . | . | . |
| . | . | . | . | . | . | . | . |
| . | . | . | . | . | . | . | . |

**References**
Whitehead, John. 1993. 'Sample Size Calculations for Ordered Categorical Data.' Statistics in Medicine, 12,
  2257-2271.
Julious, Steven A. 2010. Sample Sizes for Clinical Trials. Chapman & Hall/CRC. Boca Raton, Fl.
Machin, D., Campbell, M., Fayers, P., and Pinol, A. 1997. Sample Size Tables for Clinical Studies, 2nd
  Edition. Blackwell Science. Malden, Mass.

**Report Definitions**
Power is the probability of rejecting a false null hypothesis.
N1 and N2 are the number of subjects in the first and second groups.
N is the total sample size, N1 + N2.
θ is log odds ratio = log[odds(experimental)/odds(control)].
PC(1) Control Cat. 1 Prop. is the proportion or probability of the first (best) category of the control group.
PE(1) Trt Cat. 1 Prop. is the proportion or probability of the first (best) category of the treatment group.
Alpha is the probability of rejecting a true null hypothesis.

**Summary Statements**
Samples of 30 subjects in the control group and 30 subjects in experimental group achieve 17%
power to detect a change in the log odds ratio (θ) of 0.5000 when the significance level
(alpha) is 0.0500 using a two-sided test.

This report shows the numeric results of this power study. Following are the definitions of the columns of the report.

## Power

This is the probability of rejecting a false null hypothesis.

## N1, N2

This is the number of subjects in the control group and experimental group, respectively.

## Log Odds Ratio

This is the log of the odds ratio. It is calculated using the formula

$$\theta = \log\left\{\frac{\dfrac{Q_{iE}}{1-Q_{iE}}}{\dfrac{Q_{iC}}{1-Q_{iC}}}\right\} \quad i = 1,\ldots,K-1.$$

## Alpha

This is the probability of rejecting a true null hypothesis. This is often called the significance level.

## Beta

This is the probability of accepting a false null hypothesis.

## Control Cat. 1 Prob. PC(1)

This is the value of $p_{1C}$, the probability of a control group subject responding in category 1.

## Exp'l Cat. 1 Prob. PC(1)

This is the value of $p_{1E}$, the probability of an experimental group subject responding in category 1.

## Category Probability Distribution

**Category Probability Distribution**

| θ | Pr(1) | Pr(2) | Pr(3) | Pr(4) |
|---|-------|-------|-------|-------|
| 0.000000 | 0.2000 | 0.5000 | 0.2000 | 0.1000 |
| 0.500000 | 0.2919 | 0.5018 | 0.1432 | 0.0631 |
| 1.000000 | 0.4046 | 0.4592 | 0.0969 | 0.0393 |
| 1.500000 | 0.5284 | 0.3843 | 0.0631 | 0.0242 |
| 2.000000 | 0.6488 | 0.2964 | 0.0400 | 0.0148 |

This report shows the individual response probabilities. The first row contains the results for the control group where $\theta$ is zero. The next row gives the values of $p_{iE}$ for each value of $\theta$. The goal of the report is to let you study the impact on the $p_{iE}$ of each value of $\theta$. You can make this assessment by watching how much these values change over the corresponding value in the first row.

## Plots Section
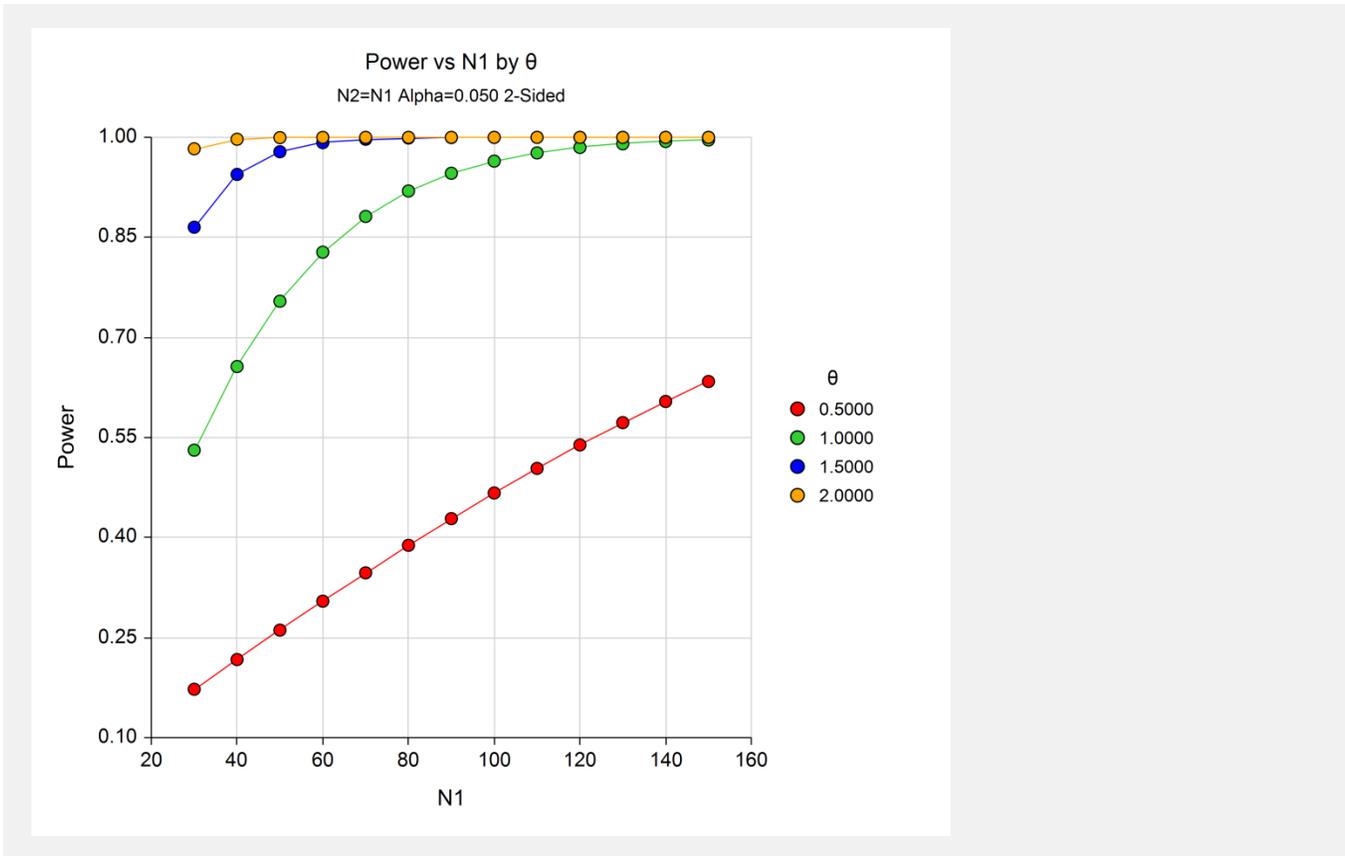
## Tests for Two Ordered Categorical Variables
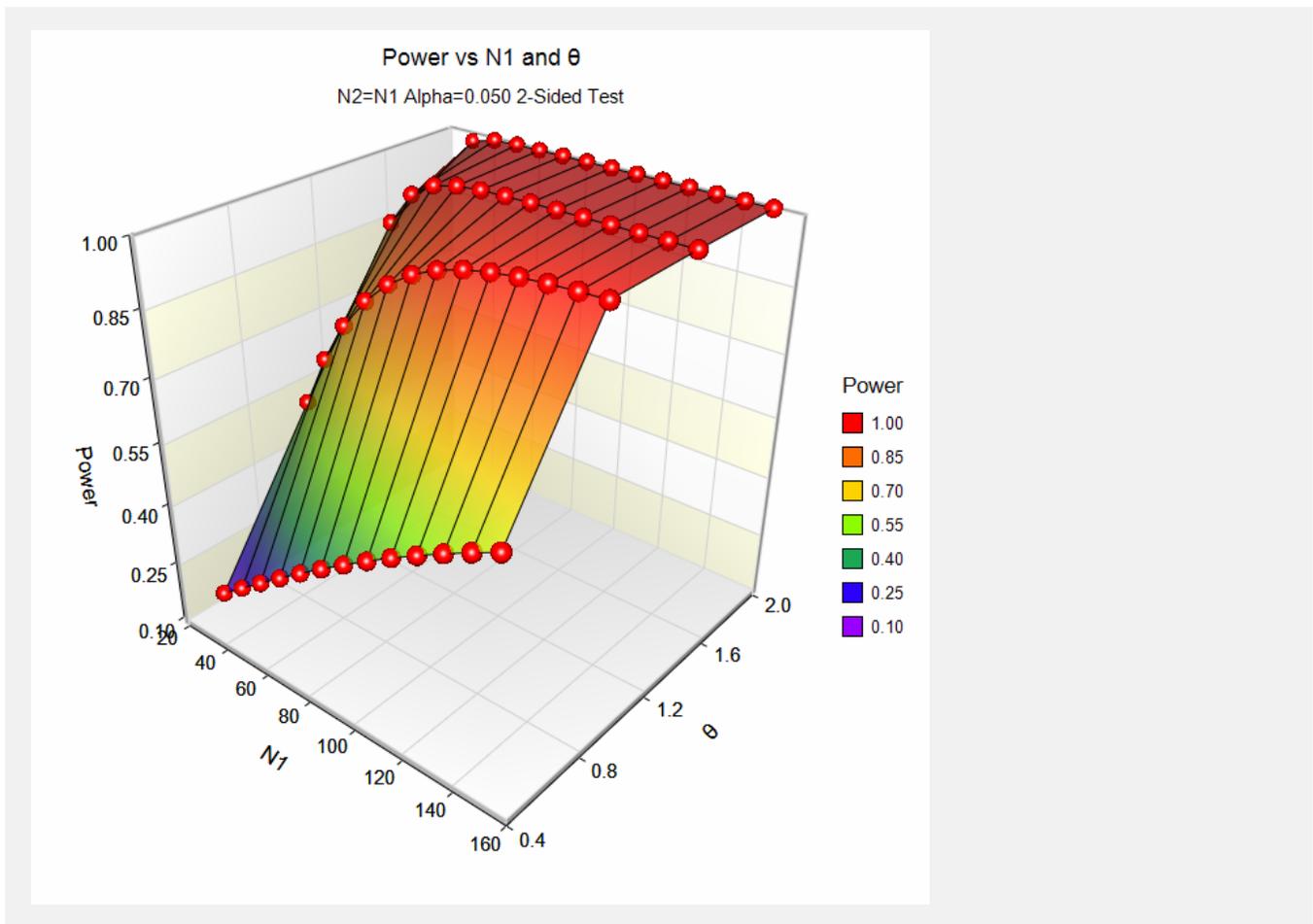


This plot gives a visual presentation to the results in the Numeric Report.

# Example 2 – Finding the Sample Size

Continuing with Example 1, the researchers want to find the sample size necessary to achieve 90% power when $\theta$ is 0.9.

## Setup

This section presents the values of each of the parameters needed to run this example. First, from the PASS Home window, load the **Tests for Two Ordered Categorical Variables** procedure window by expanding **Proportions**, then clicking on **Ordered Categorical**, and then clicking on **Tests for Two Ordered Categorical Variables**. You may then make the appropriate entries as listed below, or open **Example 2** by going to the **File** menu and choosing **Open Example Template**.

| Option | Value |
|---|---|
| **Design Tab** | |
| Solve For ................................................ | **Sample Size** |
| Null Hypothesis ...................................... | **Two-Sided** |
| Power ...................................................... | **0.9** |
| Alpha ....................................................... | **0.05** |
| Group Allocation ..................................... | **Equal (N1 = N2)** |
| Control Group Category Proportions ...... | **20 50 20 10** |
| θ (Log Odds Ratio of Treatment Group) . | **0.9** |

## Output

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

### Numeric Results

**Numeric Results for Two-Sample, Ordered-Categorical Test**
Null Hypothesis:  θ = 0    Alternative Hypothesis: θ ≠ 0

| Power | N1 | N2 | N | Trt Log Odds Ratio θ | Control Categ. 1 Prop. PC(1) | Trt Categ. 1 Prop. PE(1) | Alpha |
|---|---|---|---|---|---|---|---|
| 0.9000 | 92 | 92 | 184 | 0.9000 | 0.2000 | 0.3808 | 0.050 |

The required sample size is 92 in each group.

# Example 3 – Validation using Whitehead (1993)

Whitehead (1993) has an example in which he calculates the sample size to be 94 when $\theta$ is 0.887, alpha is 0.05, power is 90%, the control group proportions are 0.2, 0.5, 0.2, and 0.1.

## Setup

This section presents the values of each of the parameters needed to run this example. First, from the PASS Home window, load the **Tests for Two Ordered Categorical Variables** procedure window by expanding **Proportions**, then clicking on **Ordered Categorical**, and then clicking on **Tests for Two Ordered Categorical Variables**. You may then make the appropriate entries as listed below, or open **Example 3** by going to the **File** menu and choosing **Open Example Template**.

| Option | Value |
|---|---|
| **Design Tab** | |
| Solve For ................................................ | **Sample Size** |
| Null Hypothesis...................................... | **Two-Sided** |
| Power....................................................... | **0.9** |
| Alpha........................................................ | **0.05** |
| Group Allocation .................................... | **Equal (N1 = N2)** |
| Control Group Category Proportions ...... | **20 50 20 10** |
| θ (Log Odds Ratio of Treatment Group). | **0.887** |

## Output

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

### Numeric Results

**Numeric Results for Two-Sample, Ordered-Categorical Test**
Null Hypothesis: θ = 0    Alternative Hypothesis: θ ≠ 0

| Power | N1 | N2 | N | Trt Log Odds Ratio θ | Control Categ. 1 Prop. PC(1) | Trt Categ. 1 Prop. PE(1) | Alpha |
|---|---|---|---|---|---|---|---|
| 0.9000 | 94 | 94 | 189 | 0.8870 | 0.2000 | 0.3777 | 0.050 |

PASS matches the required sample size of 94 per group.