Chapter 197

# Superiority by a Margin Tests for the Odds Ratio of Two Proportions

## Introduction

This module provides power analysis and sample size calculation for superiority by a margin tests of the odds ratio in two-sample designs in which the outcome is binary. Users may choose between two popular test statistics commonly used for running the hypothesis test.

The power calculations assume that independent, random samples are drawn from two populations.

## Example

A superiority by a margin test example will set the stage for the discussion of the terminology that follows. Suppose that the current treatment for a disease works 70% of the time. A promising new treatment has been developed to the point where it can be tested. The researchers wish to show that the new treatment is better than the current treatment by at least some amount. In other words, does a clinically significant higher number of treated subjects respond to the new treatment?

Clinicians want to demonstrate the new treatment is superior to the current treatment. They must determine, however, how much more effective the new treatment must be to be adopted. Should it be adopted if 71% respond? 72%? 75%? 80%? There is a percentage above 70% at which the difference between the two treatments is no longer considered ignorable. After thoughtful discussion with several clinicians, it was decided that if the odds ratio of new treatment to reference is at least 1.1, the new treatment would be adopted. This ratio is called the *margin of superiority*. The margin of superiority in this example is 1.1.

The developers must design an experiment to test the hypothesis that the odds ratio of the new treatment to the reference is at least 1.1. The statistical hypothesis to be tested is

$$H_0: O_1/O_2 \le 1.1 \quad \text{versus} \quad H_1: O_1/O_2 > 1.1$$

Notice that when the null hypothesis is rejected, the conclusion is that the odds ratio is at least 1.1. Note that even though the response rate of the current treatment is 0.70, the hypothesis test is about an odds ratio of 1.1. Also notice that a rejection of the null hypothesis results in the conclusion of interest.

# Technical Details

The details of sample size calculation for the two-sample design for binary outcomes are presented in the chapter "Tests for Two Proportions," and they will not be duplicated here. Instead, this chapter only discusses those changes necessary for superiority by a margin tests.

This procedure has the capability for calculating power based on large sample (normal approximation) results and based on the enumeration of all possible values in the binomial distribution.

Suppose you have two populations from which dichotomous (binary) responses will be recorded. Assume without loss of generality that the higher proportions are better. The probability (or risk) of cure in population 1 (the treatment group) is $p_1$ and in population 2 (the reference group) is $p_2$. Random samples of $n_1$ and $n_2$ individuals are obtained from these two populations. The data from these samples can be displayed in a 2-by-2 contingency table as follows

| Group | Success | Failure | Total |
|---|---|---|---|
| **Treatment** | $x_{11}$ | $x_{12}$ | $n_1$ |
| **Control** | $x_{21}$ | $x_{22}$ | $n_2$ |
| **Totals** | $m_1$ | $m_2$ | $N$ |

The binomial proportions, $p_1$ and $p_2$, are estimated from these data using the formulae

$$\hat{p}_1 = \frac{a}{m} = \frac{x_{11}}{n_1} \text{ and } \hat{p}_2 = \frac{b}{n} = \frac{x_{21}}{n_2}$$

Define $O_i = p_i/(1 - p_i)$. Let $p_{1.0}$ represent the group 1 proportion tested by the null hypothesis, $H_0$. The power of a test is computed at a specific value of the proportion which we will call $p_{1.1}$. Let $\psi_0$ represent the smallest odds ratio (margin of superiority) between the two proportions that still results in the conclusion that the new treatment is superior to the current treatment. For a superiority by a margin test, $\psi_0 > 1$ The set of statistical hypotheses that are tested is

$$H_0: O_1/O_2 \le \psi_0 \quad \text{versus} \quad H_1: O_1/O_2 > \psi_0$$

which can be rearranged to give

$$H_0: O_1 \le O_2\psi_0 \quad \text{versus} \quad H_1: O_1 > O_2\psi_0$$

There are three common methods of specifying the margin of superiority. The most direct is to simply give values for $p_2$ and $p_{1.0}$. However, it is often more meaningful to give $p_2$ and then specify $p_{1.0}$ implicitly by specifying the difference, ratio, or odds ratio. Mathematically, the definitions of these parameterizations are

| Parameter | Computation | Hypotheses |
|---|---|---|
| Difference | $\delta_0 = p_{1.0} - p_2$ | $H_0: p_1 - p_2 \le \delta_0 \quad \text{versus} \quad H_1: p_1 - p_2 > \delta_0$ |
| Ratio | $\phi_0 = p_{1.0}/p_2$ | $H_0: p_1/p_2 \le \phi_0 \quad \text{versus} \quad H_1: p_1/p_2 > \phi_0$ |
| Odds Ratio | $\psi_0 = O_{1.0}/O_2$ | $H_0: O_1/O_2 \le \psi_0 \quad \text{versus} \quad H_1: O_1/O_2 > \psi_0$ |

## Odds Ratio

The odds ratio, $\psi = O_1/O_2 = (p_1/(1-p_1))/(p_2/(1-p_2))$, gives the relative change in the odds of the response. Testing superiority by a margin uses the formulation

$$H_0: O_1/O_2 \leq \psi_0 \quad \text{versus} \quad H_1: O_1/O_2 > \psi_0$$

or equivalently

$$H_0: \psi \leq \psi_0 \quad \text{versus} \quad H_1: \psi > \psi_0.$$

For superiority by a margin tests with higher proportions better, $\psi_0 > 1$. For superiority by a margin tests with higher proportions worse, $\psi_0 < 1$.

## A Note on Setting the Significance Level, Alpha

Setting the significance level has always been somewhat arbitrary. For planning purposes, the standard has become to set alpha to 0.05 for two-sided tests. Almost universally, when someone states that a result is statistically significant, they mean statistically significant at the 0.05 level.

Although 0.05 may be the standard for two-sided tests, it is not always the standard for one-sided tests, such as superiority by a margin tests. Statisticians often recommend that the alpha level for one-sided tests be set at 0.025 since this is the amount put in each tail of a two-sided test.

## Power Calculation

The power for a test statistic that is based on the normal approximation can be computed exactly using two binomial distributions. The following steps are taken to compute the power of these tests.

1. Find the critical value using the standard normal distribution. The critical value, $z_{critical}$, is that value of $z$ that leaves exactly the target value of alpha in the appropriate tail of the normal distribution.

2. Compute the value of the test statistic, $z_t$, for every combination of $x_{11}$ and $x_{21}$. Note that $x_{11}$ ranges from 0 to $n_1$, and $x_{21}$ ranges from 0 to $n_2$. A small value (around 0.0001) can be added to the zero-cell counts to avoid numerical problems that occur when the cell value is zero.

3. If $z_t > z_{critical}$, the combination is in the rejection region. Call all combinations of $x_{11}$ and $x_{21}$ that lead to a rejection the set $A$.

4. Compute the power for given values of $p_{1.1}$ and $p_2$ as

$$1 - \beta = \sum_A \binom{n_1}{x_{11}} p_{1.1}^{x_{11}} q_{1.1}^{n_1-x_{11}} \binom{n_2}{x_{21}} p_2^{x_{21}} q_2^{n_2-x_{21}}.$$

5. Compute the actual value of alpha achieved by the design by substituting $p_{1.0}$ for $p_{1.1}$ to obtain

$$\alpha^* = \sum_A \binom{n_1}{x_{11}} p_{1.0}^{x_{11}} q_{1.0}^{n_1-x_{11}} \binom{n_2}{x_{21}} p_2^{x_{21}} q_2^{n_2-x_{21}}.$$

## Asymptotic Approximations

When the values of $n_1$ and $n_2$ are large (say over 200), these formulas often take a long time to evaluate. In this case, a large sample approximation can be used. The large sample approximation is made by replacing the values of $\hat{p}_1$ and $\hat{p}_2$ in the $z$ statistic with the corresponding values of $p_{1.1}$ and $p_2$, and then computing the results based on the normal distribution.

# Test Statistics

Two test statistics have been proposed for testing whether the odds ratio is different from a specified value. The main difference between the test statistics is in the formula used to compute the standard error used in the denominator. These tests are both likelihood score tests.

In power calculations, the values of $\hat{p}_1$ and $\hat{p}_2$ are not known. The corresponding values of $p_{1.1}$ and $p_2$ may be reasonable substitutes.

Following is a list of the test statistics available in **PASS**. The availability of several test statistics begs the question of which test statistic one should use. The answer is simple: <u>one should use the test statistic that will be used to analyze the data</u>. You may choose a method because it is a standard in your industry, because it seems to have better statistical properties, or because your statistical package calculates it. Whatever your reasons for selecting a certain test statistic, you should use the same test statistic when doing the analysis after the data have been collected.

## Miettinen and Nurminen's Likelihood Score Test

Miettinen and Nurminen (1985) proposed a test statistic for testing whether the odds ratio is equal to a specified value, $\psi_0$. Because the approach they used with the difference and ratio does not easily extend to the odds ratio, they used a score statistic approach for the odds ratio. The regular MLE's are $\hat{p}_1$ and $\hat{p}_2$. The constrained MLE's are $\tilde{p}_1$ and $\tilde{p}_2$. These estimates are constrained so that $\tilde{\psi} = \psi_0$. A correction factor of $N/(N\text{-}1)$ is applied to make the variance estimate less biased. The significance level of the test statistic is based on the asymptotic normality of the score statistic.

The formula for computing the test statistic is

$$z_{MNO} = \frac{\dfrac{(\hat{p}_1 - \tilde{p}_1)}{\tilde{p}_1 \tilde{q}_1} - \dfrac{(\hat{p}_2 - \tilde{p}_2)}{\tilde{p}_2 \tilde{q}_2}}{\sqrt{\left(\dfrac{1}{n_1 \tilde{p}_1 \tilde{q}_1} + \dfrac{1}{n_2 \tilde{p}_2 \tilde{q}_2}\right)\left(\dfrac{N}{N-1}\right)}}$$

where

$$\tilde{p}_1 = \frac{\tilde{p}_2 \psi_0}{1 + \tilde{p}_2(\psi_0 - 1)}$$

$$\tilde{p}_2 = \frac{-B + \sqrt{B^2 - 4AC}}{2A}$$

$$A = n_2(\psi_0 - 1),$$

$$B = n_1\psi_0 + n_2 - m_1(\psi_0 - 1),$$

$$C = -m_1$$

## Farrington and Manning's Likelihood Score Test

Farrington and Manning (1990) indicate that the Miettinen and Nurminen statistic may be modified by removing the factor $N/(N\text{-}1)$.

The formula for computing this test statistic is

$$z_{FMO} = \frac{\frac{(\hat{p}_1 - \tilde{p}_1)}{\tilde{p}_1\tilde{q}_1} - \frac{(\hat{p}_2 - \tilde{p}_2)}{\tilde{p}_2\tilde{q}_2}}{\sqrt{\left(\frac{1}{n_1\tilde{p}_1\tilde{q}_1} + \frac{1}{n_2\tilde{p}_2\tilde{q}_2}\right)}}$$

where the estimates $\tilde{p}_1$ and $\tilde{p}_2$ are computed as in the corresponding test of Miettinen and Nurminen (1985) given above.

# Example 1 – Finding Power

A study is being designed to establish the superiority of a new treatment compared to the current treatment. Historically, the current treatment has enjoyed a 62.5% cure rate. The new treatment is hoped to perform better than the current treatment. Thus, the new treatment will be adopted if it is more effective than the current treatment by a clinically significant amount. The researchers will recommend adoption of the odds ratio of the new treatment to the old treatment is at least 1.5.

The researchers plan to use the Farrington and Manning likelihood score test statistic to analyze the data that will be (or has been) obtained. They want to study the power of the Farrington and Manning test at group sample sizes ranging from 50 to 500 when the actual odds ratio is 2.0. The significance level will be 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 ......................................................**Power**
Power Calculation Method............................**Normal Approximation**
Higher Proportions Are .................................**Better (H1: OR > OR0)**
Test Type......................................................**Likelihood Score (Farr. & Mann.)**
Alpha............................................................**0.05**
Group Allocation ..........................................**Equal (N1 = N2)**
Sample Size Per Group ................................**50 to 500 by 50**
OR0 (Superiority Odds Ratio).......................**1.5**
OR1 (Actual Odds Ratio) ..............................**2.0**
P2 (Group 2 Proportion)................................**0.625**

# Output

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

## Numeric Reports

**Numeric Results**
───────────────────────────────────────────────────────────────────────────────
Solve For:        Power
Groups:           1 = Treatment, 2 = Reference
Test Statistic:   Farrington & Manning Likelihood Score Test
Hypotheses:       H0: OR ≤ OR0   vs.   H1: OR > OR0
───────────────────────────────────────────────────────────────────────────────

| | Sample Size | | | Proportions | | | Odds Ratio | | |
|---|---|---|---|---|---|---|---|---|---|
| Power* | N1 | N2 | N | Superiority P1.0 | Actual P1.1 | Reference P2 | Superiority OR0 | Actual OR1 | Alpha |
| 0.16278 | 50 | 50 | 100 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |
| 0.23613 | 100 | 100 | 200 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |
| 0.30292 | 150 | 150 | 300 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |
| 0.36502 | 200 | 200 | 400 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |
| 0.42291 | 250 | 250 | 500 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |
| 0.47676 | 300 | 300 | 600 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |
| 0.52669 | 350 | 350 | 700 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |
| 0.57279 | 400 | 400 | 800 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |
| 0.61522 | 450 | 450 | 900 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |
| 0.65413 | 500 | 500 | 1000 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |

* Power was computed using the normal approximation method.

| | |
|---|---|
| Power | The probability of rejecting a false null hypothesis when the alternative hypothesis is true. |
| N1 and N2 | The number of items sampled from each population. |
| N | The total sample size. N = N1 + N2. |
| P1 | The proportion for group 1, which is the treatment or experimental group. |
| P1.0 | The smallest group 1 proportion that still yields a superiority conclusion. P1.0 = P1|H0. |
| P1.1 | The proportion for group 1 used for the alternative hypothesis, H1. P1.1 = P1|H1. |
| P2 | The proportion for group 2, which is the standard, reference, or control group. |
| OR0 | The superiority odds ratio, [P1/(1-P1)] / [P2/(1-P2)], assuming H0. |
| OR1 | The superiority odds ratio, [P1/(1-P1)] / [P2/(1-P2)], assuming H1. |
| Alpha | The probability of rejecting a true null hypothesis. |

**Summary Statements**
───────────────────────────────────────────────────────────────────────────────
A parallel two-group design will be used to test whether the Group 1 (treatment) proportion (P1) is superior to the Group 2 (reference) proportion (P2) by a margin, with a superiority odds ratio of 1.5 (H0: OR ≤ 1.5 versus H1: OR > 1.5). The comparison will be made using a one-sided, two-sample Score test (Farrington & Manning) with a Type I error rate (α) of 0.05. The reference group proportion is assumed to be 0.625. To detect an odds ratio (O1 / O2) of 2 (or P1 of 0.7692) with sample sizes of 50 for the treatment group and 50 for the reference group, the power is 0.16278.
───────────────────────────────────────────────────────────────────────────────

**Dropout-Inflated Sample Size**

| Dropout Rate | Sample Size | | | Dropout-Inflated Enrollment Sample Size | | | Expected Number of Dropouts | | |
|---|---|---|---|---|---|---|---|---|---|
| | N1 | N2 | N | N1' | N2' | N' | D1 | D2 | D |
| 20% | 50 | 50 | 100 | 63 | 63 | 126 | 13 | 13 | 26 |
| 20% | 100 | 100 | 200 | 125 | 125 | 250 | 25 | 25 | 50 |
| 20% | 150 | 150 | 300 | 188 | 188 | 376 | 38 | 38 | 76 |
| 20% | 200 | 200 | 400 | 250 | 250 | 500 | 50 | 50 | 100 |
| 20% | 250 | 250 | 500 | 313 | 313 | 626 | 63 | 63 | 126 |
| 20% | 300 | 300 | 600 | 375 | 375 | 750 | 75 | 75 | 150 |
| 20% | 350 | 350 | 700 | 438 | 438 | 876 | 88 | 88 | 176 |
| 20% | 400 | 400 | 800 | 500 | 500 | 1000 | 100 | 100 | 200 |
| 20% | 450 | 450 | 900 | 563 | 563 | 1126 | 113 | 113 | 226 |
| 20% | 500 | 500 | 1000 | 625 | 625 | 1250 | 125 | 125 | 250 |

| | |
|---|---|
| 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. |
| N1, N2, and N | The evaluable sample sizes at which power is computed (as entered by the user). If N1 and N2 subjects are evaluated out of the N1' and N2' subjects that are enrolled in the study, the design will achieve the stated power. |
| N1', N2', and N' | The number of subjects that should be enrolled in the study in order to obtain N1, N2, and N evaluable subjects, based on the assumed dropout rate. N1' and N2' are calculated by inflating N1 and N2 using the formulas N1' = N1 / (1 - DR) and N2' = N2 / (1 - DR), with N1' and N2' 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.) |
| D1, D2, and D | The expected number of dropouts. D1 = N1' - N1, D2 = N2' - N2, and D = D1 + D2. |

**Dropout Summary Statements**

Anticipating a 20% dropout rate, 63 subjects should be enrolled in Group 1, and 63 in Group 2, to obtain final group sample sizes of 50 and 50, respectively.

**References**

Chow, S.C., Shao, J., and Wang, H. 2008. Sample Size Calculations in Clinical Research, Second Edition. Chapman & Hall/CRC. Boca Raton, Florida.

Farrington, C. P. and Manning, G. 1990. 'Test Statistics and Sample Size Formulae for Comparative Binomial Trials with Null Hypothesis of Non-Zero Risk Difference or Non-Unity Relative Risk.' Statistics in Medicine, Vol. 9, pages 1447-1454.

Fleiss, J. L., Levin, B., Paik, M.C. 2003. Statistical Methods for Rates and Proportions. Third Edition. John Wiley & Sons. New York.

Gart, John J. and Nam, Jun-mo. 1988. 'Approximate Interval Estimation of the Ratio in Binomial Parameters: A Review and Corrections for Skewness.' Biometrics, Volume 44, Issue 2, 323-338.

Gart, John J. and Nam, Jun-mo. 1990. 'Approximate Interval Estimation of the Difference in Binomial Parameters: Correction for Skewness and Extension to Multiple Tables.' Biometrics, Volume 46, Issue 3, 637-643.

Julious, S. A. and Campbell, M. J. 2012. 'Tutorial in biostatistics: sample sizes for parallel group clinical trials with binary data.' Statistics in Medicine, 31:2904-2936.

Lachin, John M. 2000. Biostatistical Methods. John Wiley & Sons. New York.

Machin, D., Campbell, M., Fayers, P., and Pinol, A. 1997. Sample Size Tables for Clinical Studies, 2nd Edition. Blackwell Science. Malden, Mass.
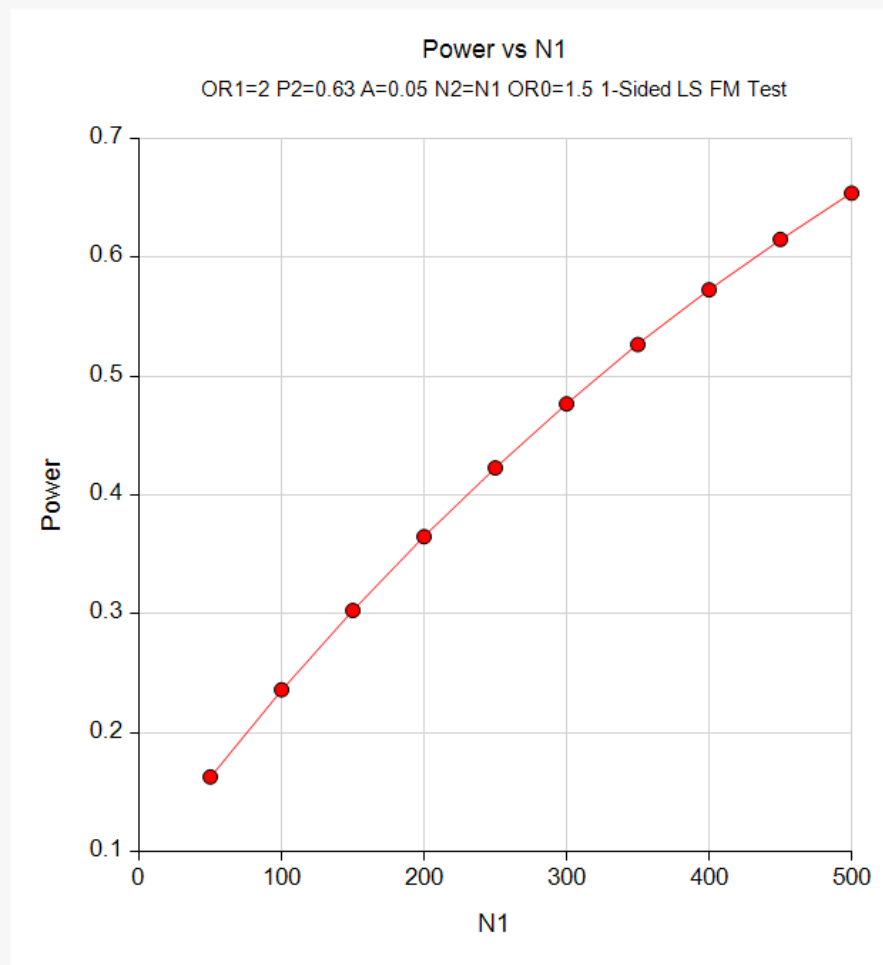
Miettinen, O.S. and Nurminen, M. 1985. 'Comparative analysis of two rates.' Statistics in Medicine 4: 213-226.

This report shows the values of each of the parameters, one scenario per row.

# Plots Section

**Plots**

_____



The values from the table are displayed in the above chart. These charts give us a quick look at the sample size that will be required for various sample sizes.

# Example 2 – Finding the Sample Size

Continuing with the scenario given in Example 1, the researchers want to determine the sample size necessary to achieve a power of 0.80.

## 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**
Power Calculation Method............................**Normal Approximation**
Higher Proportions Are ................................**Better (H1: OR > OR0)**
Test Type....................................................**Likelihood Score (Farr. & Mann.)**
Power.........................................................**0.80**
Alpha.........................................................**0.05**
Group Allocation .........................................**Equal (N1 = N2)**
OR0 (Superiority Odds Ratio).......................**1.5**
OR1 (Actual Odds Ratio) .............................**2.0**
P2 (Group 2 Proportion)...............................**0.625**

## Output

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

**Numeric Results**

Solve For:        Sample Size
Groups:          1 = Treatment, 2 = Reference
Test Statistic:   Farrington & Manning Likelihood Score Test
Hypotheses:      H0: OR ≤ OR0   vs.   H1: OR > OR0

| Power | | Sample Size | | | Proportions | | | Odds Ratio | | |
|---|---|---|---|---|---|---|---|---|---|---|
| Target | Actual* | N1 | N2 | N | Superiority P1.0 | Actual P1.1 | Reference P2 | Superiority OR0 | Actual OR1 | Alpha |
| 0.8 | 0.80002 | 745 | 745 | 1490 | 0.7143 | 0.7692 | 0.625 | 1.5 | 2 | 0.05 |

* Power was computed using the normal approximation method.

The required sample size is 1035 per group.

# Example 3 – Comparing the Power of the Two Test Statistics

Continuing with Example 2, the researchers want to determine which of the two possible test statistics to adopt by using the comparative reports and charts that **PASS** produces. They decide to compare the powers from binomial enumeration and actual alphas for various sample sizes between 1000 and 1200.

## 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 3** 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 ............................................................... | **Power** |
| Power Calculation Method ..................................... | **Binomial Enumeration** |
| Maximum N1 or N2 for Binomial Enumeration ........ | **5000** |
| Zero Count Adjustment Method ............................. | **Add to zero cells only** |
| Zero Count Adjustment Value ................................ | **0.0001** |
| Higher Proportions Are ......................................... | **Better (H1: OR > OR0)** |
| Test Type............................................................... | **Likelihood Score (Farr. & Mann.)** |
| Alpha...................................................................... | **0.05** |
| Group Allocation ................................................... | **Equal (N1 = N2)** |
| Sample Size Per Group ......................................... | **600 700 800** |
| OR0 (Superiority Odds Ratio) ................................ | **1.5** |
| OR1 (Actual Odds Ratio) ....................................... | **2.0** |
| P2 (Group 2 Proportion).......................................... | **0.625** |

Reports Tab

| | |
|---|---|
| Show Comparative Reports ................................... | **Checked** |

Comparative Plots Tab

| | |
|---|---|
| Show Comparative Plots......................................... | **Checked** |

# Output

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

**Power Comparison of Two Different Tests**

Hypotheses:   H0: OR ≤ OR0   vs.   H1: OR > OR0

| Sample Size | | | | | | | Power | |
| N1 | N2 | N | P2 | OR0 | OR1 | Target Alpha | F.M. Score | M.N. Score |
|---|---|---|---|---|---|---|---|---|
| 600 | 600 | 1200 | 0.625 | 1.5 | 2 | 0.05 | 0.7297 | 0.7297 |
| 700 | 700 | 1400 | 0.625 | 1.5 | 2 | 0.05 | 0.7862 | 0.7862 |
| 800 | 800 | 1600 | 0.625 | 1.5 | 2 | 0.05 | 0.8322 | 0.8313 |

Note: Power was computed using binomial enumeration of all possible outcomes.

**Actual Alpha Comparison of Two Different Tests**

Hypotheses:   H0: OR ≤ OR0   vs.   H1: OR > OR0

| Sample Size | | | | | | | Alpha | |
| N1 | N2 | N | P2 | OR0 | OR1 | Target | F.M. Score | M.N. Score |
|---|---|---|---|---|---|---|---|---|
| 600 | 600 | 1200 | 0.625 | 1.5 | 2 | 0.05 | 0.0503 | 0.0503 |
| 700 | 700 | 1400 | 0.625 | 1.5 | 2 | 0.05 | 0.0502 | 0.0502 |
| 800 | 800 | 1600 | 0.625 | 1.5 | 2 | 0.05 | 0.0502 | 0.0501 |

Note: Actual alpha was computed using binomial enumeration of all possible outcomes.

**Plots**
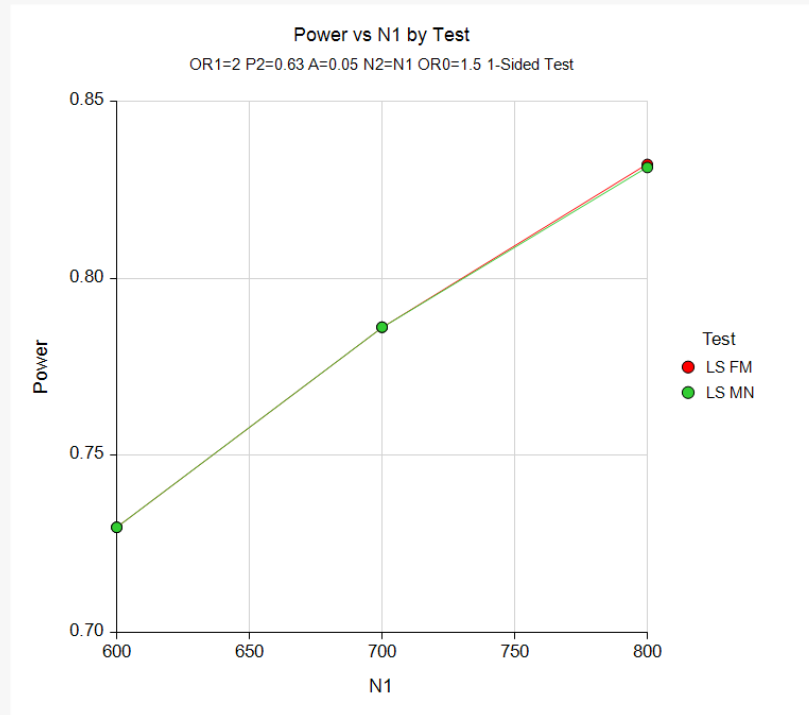


The power is almost exactly the same for both tests, which is not surprising given the large sample size.

# Example 4 – Comparing Power Calculation Methods

Continuing with Example 3, let's see how the results compare if we were to use approximate power calculations instead of power calculations based on binomial enumeration.

## 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 4** 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 ....................................................**Power**
Power Calculation Method............................**Normal Approximation**
Higher Proportions Are ................................**Better (H1: OR > OR0)**
Test Type....................................................**Likelihood Score (Farr. & Mann.)**
Alpha..........................................................**0.05**
Group Allocation .........................................**Equal (N1 = N2)**
Sample Size Per Group ...............................**600 700 800**
OR0 (Superiority Odds Ratio).......................**1.5**
OR1 (Actual Odds Ratio) ..............................**2.0**
P2 (Group 2 Proportion)...............................**0.625**

Reports Tab

Show Power Detail Report............................**Checked**

## Output

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

**Power Detail Report**

Test Statistic: Farrington & Manning Likelihood Score Test
Hypotheses: H0: OR ≤ OR0   vs.   H1: OR > OR0

| Sample Size | | | | | | Normal Approximation | | Binomial Enumeration | |
|---|---|---|---|---|---|---|---|---|---|
| N1 | N2 | N | P2 | OR0 | OR1 | Power | Alpha | Power | Alpha |
| 600 | 600 | 1200 | 0.625 | 1.5 | 2 | 0.72209 | 0.05 | 0.72971 | 0.05 |
| 700 | 700 | 1400 | 0.625 | 1.5 | 2 | 0.77821 | 0.05 | 0.78622 | 0.05 |
| 800 | 800 | 1600 | 0.625 | 1.5 | 2 | 0.82407 | 0.05 | 0.83218 | 0.05 |

Notice that the approximate power values are close to the binomial enumeration values for all sample sizes.

# Example 5 – Validation

We could not find a validation example for a superiority by a margin test for the odds ratio of two proportions. The calculations are basically the same as those for a superiority by a margin test of the ratio of two proportions, which has been validated using Blackwelder (1993). We refer you to Example 5 of Chapter 196, "Superiority by a Margin Tests for the Ratio of Two Proportions," for a validation example.