Chapter 206

Non-Unity Null Tests for the Ratio of Two Proportions

Introduction

This module computes power and sample size for hypothesis tests of the ratio of two independent proportions where the null-hypothesized value is not equal to one. The *non-offset* case is available in another procedure. This procedure compares the power achieved by each of several test statistics.

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

Technical Details

Suppose you have two populations from which dichotomous (binary) responses will be recorded. The probability (or risk) of obtaining the event of interest in population 1 (the treatment group) is p_1 and in population 2 (the control group) is p_2. The corresponding failure proportions are given by $q_1 = 1 - p_1$ and $q_2 = 1 - p_2$.

An assumption is made that the responses from each group follow a binomial distribution. This means that the event probability, p_i, is the same for all subjects within the group and that the response from one subject is independent of that of any other subject.

Random samples of *m* and *n* 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	*a*	*c*	*m*
Control	*b*	*d*	*n*
Total	*s*	*f*	*N*

The following alternative notation is sometimes used.

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

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

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

Comparing Two Proportions

When analyzing studies such as this, you usually want to compare the two binomial probabilities, p_1 and p_2. The most direct method of comparing these quantities is to calculate their difference or their ratio. If the binomial probability is expressed in terms of odds rather than probability, another measure is the odds ratio. Mathematically, these comparison parameters are

Parameter	**Computation**
Difference	$\delta = p_1 - p_2$
Risk Ratio	$\phi = p_1 / p_2$
Odds Ratio	$\psi = \frac{p_1/(1-p_1)}{p_2/(1-p_2)} = \frac{p_1 q_2}{p_2 q_1}$

The choice of which of these measures is used might seem arbitrary, but it is not. Not only will the interpretation be different, but, for small sample sizes, the powers of tests based on different parameters will be different. The non-null case is commonly used in equivalence and non-inferiority testing.

Ratio

The (risk) ratio, $\phi = p_1 / p_2$, gives the relative change in the disease risk due to the application of the treatment. This parameter is also direct and easy to interpret. To compare this with the difference, consider a treatment that reduces the risk of disease from 0.1437 to 0.0793. One should consider which single number is more enlightening, the fact that the absolute risk of disease has been decreased by 0.0644, or the fact that risk of disease in the treatment group is only 55.18% of that in the control group. In many cases, the percentage (risk ratio) communicates the impact of the treatment better than the absolute change.

Perhaps the biggest drawback to this parameter is that it cannot be calculated in one of the most common experimental designs, the case-control study. Another drawback is that the odds ratio occurs directly in the likelihood equations and as a parameter in logistic regression.

Hypothesis Tests

Although several statistical tests are available for testing the inequality of two proportions, only a few can be generalized to the non-null case. No single test is the champion in every situation, so one should compare the powers of the various tests to determine which to use.

Ratio

The (risk) ratio, $\phi = p_1 / p_2$, is often preferred as a comparison parameter because it expresses the difference as a percentage rather than an amount. Three sets of statistical hypotheses can be formulated:

1. $H_0{:}\, p_1 / p_2 = \phi_0$ versus $H_1{:}\, p_1 / p_2 \neq \phi_0$; this is often called the *two-tailed test*.
2. $H_0{:}\, p_1 / p_2 \leq \phi_0$ versus $H_0{:}\, p_1 / p_2 > \phi_0$; this is often called the *upper-tailed test*.
3. $H_0{:}\, p_1 / p_2 \geq \phi_0$ versus $H_0{:}\, p_1 / p_2 < \phi_0$; this is often called the *lower-tailed 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 such a test.

1. Find the critical value (or values in the case of a two-sided test) 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. For example, for an upper-tailed test with a target alpha of 0.05, the critical value is 1.645.
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 $p_{1.1}$ (p_1 under the alternative) 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 $p_{1.0}$ (p_1 under the null) 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 is used. The large sample approximation is made by replacing the values of $\hat{p}_1$ and $\hat{p}_2$ in the *z* values with the corresponding values of p_1 and p_2 under the alternative hypothesis and then computing the results based on the normal distribution. Note that in large samples, the Farrington and Manning statistic is substituted for the Gart and Nam statistic.

Test Statistics

Several test statistics have been proposed for testing whether the difference, ratio, or odds ratio are different from a specified value. The main difference among the several test statistics is in the formula used to compute the standard error used in the denominator. These tests are based on the following *z*-test

$$z_t = \frac{\hat{p}_1 - \hat{p}_2 - \delta_0 - c}{\hat{\sigma}}$$

The constant, *c*, represents a continuity correction that is applied in some cases. When the continuity correction is not used, *c* is zero. In power calculations, the values of $\hat{p}_1$ and $\hat{p}_2$ are not known. The corresponding values of p_1 and p_2 under the alternative hypothesis are 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 you should use. The answer is simple: you should use the test statistic that you will use to analyze your data. 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 during power or sample calculations.

Miettinen and Nurminen's Likelihood Score Test

Miettinen and Nurminen (1985) proposed a test statistic for testing whether the ratio is equal to a specified value, ϕ_0. The regular MLE's, $\hat{p}_1$ and $\hat{p}_2$, are used in the numerator of the score statistic while MLE's $\tilde{p}_1$ and $\tilde{p}_2$, constrained so that $\tilde{p}_1 / \tilde{p}_2 = \phi_0$, are used in the denominator. A correction factor of *N*/(*N*-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

$$z_{MNR} = \frac{\hat{p}_1 / \hat{p}_2 - \phi_0}{\sqrt{\left(\frac{\tilde{p}_1 \tilde{q}_1}{n_1} + \phi_0^2 \frac{\tilde{p}_2 \tilde{q}_2}{n_2}\right)\left(\frac{N}{N-1}\right)}}$$

where

$$\tilde{p}_1 = \tilde{p}_2 \phi_0$$

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

$$A = N\phi_0$$

$$B = -[n_1\phi_0 + x_{11} + n_2 + x_{21}\phi_0]$$

$$C = m_1$$

Farrington and Manning's Likelihood Score Test

Farrington and Manning (1990) proposed a test statistic for testing whether the ratio is equal to a specified value, ϕ_0. The regular MLE's, $\hat{p}_1$ and $\hat{p}_2$, are used in the numerator of the score statistic while MLE's $\tilde{p}_1$ and $\tilde{p}_2$, constrained so that $\tilde{p}_1/\tilde{p}_2 = \phi_0$, are used in the denominator. 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

$$z_{FMR} = \frac{\hat{p}_1 \,/\, \hat{p}_2 - \phi_0}{\sqrt{\left(\frac{\tilde{p}_1\tilde{q}_1}{n_1} + \phi_0^2 \frac{\tilde{p}_2\tilde{q}_2}{n_2}\right)}}$$

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

Gart and Nam's Likelihood Score Test

Gart and Nam (1988), page 329, proposed a modification to the Farrington and Manning (1988) ratio test that corrected for skewness. Let $z_{FMR}(\phi)$ stand for the Farrington and Manning ratio test statistic described above. The skewness corrected test statistic, z_{GNR}, is the appropriate solution to the quadratic equation

$$(-\tilde{\varphi})z_{GNR}^2 + (-1)z_{GNR} + (z_{FMR}(\phi) + \tilde{\varphi}) = 0$$

where

$$\tilde{\varphi} = \frac{1}{6\tilde{u}^{3/2}}\left(\frac{\tilde{q}_1(\tilde{q}_1 - \tilde{p}_1)}{n_1^2\tilde{p}_1^2} - \frac{\tilde{q}_2(\tilde{q}_2 - \tilde{p}_2)}{n_2^2\tilde{p}_2^2}\right)$$

$$\tilde{u} = \frac{\tilde{q}_1}{n_1\tilde{p}_1} + \frac{\tilde{q}_2}{n_2\tilde{p}_2}$$

Example 1 – Finding Power

A study is being designed to determine the effectiveness of a new treatment. The standard treatment has a success rate of 65%. They would like to show that the success rate ratio of the new treatment to the old treatment is at least 1.1.

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 200 for detecting a ratio of 1.1 when the actual ratio ranges from 1.2 to 1.5. The significance level will be 0.025.

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**
Alternative Hypothesis .... **One-Sided (H1: P1/P2 > R0)**
Test Type .... **Likelihood Score (Farr. & Mann.)**
Alpha... **0.025**
Group Allocation .... **Equal (N1 = N2)**
Sample Size Per Group **50 100 150 200**
R0 (Ratio|H0 = P1.0/P2) **1.1**
R1 (Ratio|H1 = P1.1/P2) **1.2 1.3 1.4 1.5**
P2 (Group 2 Proportion)................................ **0.65**

Output

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

Numeric Reports

Numeric Results

Solve For: Power
Test Statistic: Farrington & Manning Likelihood Score Test
Hypotheses: H0: P1 / P2 ≤ R0 vs. H1: P1 / P2 > R0

	Sample Size			Proportions			Ratio		
Power*	N1	N2	N	P1|H0 P1.0	P1|H1 P1.1	Reference P2	Ratio|H0 R0	Ratio|H1 R1	Alpha
0.10144	50	50	100	0.715	0.780	0.65	1.1	1.2	0.025
0.16144	100	100	200	0.715	0.780	0.65	1.1	1.2	0.025
0.22064	150	150	300	0.715	0.780	0.65	1.1	1.2	0.025
0.27900	200	200	400	0.715	0.780	0.65	1.1	1.2	0.025
0.30085	50	50	100	0.715	0.845	0.65	1.1	1.3	0.025
0.53006	100	100	200	0.715	0.845	0.65	1.1	1.3	0.025
0.70327	150	150	300	0.715	0.845	0.65	1.1	1.3	0.025
0.82128	200	200	400	0.715	0.845	0.65	1.1	1.3	0.025
0.63410	50	50	100	0.715	0.910	0.65	1.1	1.4	0.025
0.90292	100	100	200	0.715	0.910	0.65	1.1	1.4	0.025
0.97891	150	150	300	0.715	0.910	0.65	1.1	1.4	0.025
0.99597	200	200	400	0.715	0.910	0.65	1.1	1.4	0.025
0.92217	50	50	100	0.715	0.975	0.65	1.1	1.5	0.025
0.99753	100	100	200	0.715	0.975	0.65	1.1	1.5	0.025
0.99995	150	150	300	0.715	0.975	0.65	1.1	1.5	0.025
1.00000	200	200	400	0.715	0.975	0.65	1.1	1.5	0.025

* 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 proportion for group 1 under the null hypothesis.
P1.1 The proportion for group 1 under the alternative hypothesis at which power and sample size calculations are made.
P2 The proportion for group 2, which is the standard, reference, or control group.
R0 The ratio (P1/P2) under the null hypothesis.
R1 The ratio (P1/P2) under the alternative hypothesis used for power and sample size calculations.
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 different from the Group 2 (reference) proportion (P2) by a margin, with a non-unity null ratio of 1.1 (H0: P1 / P2 ≤ 1.1 versus H1: P1 / P2 > 1.1). The comparison will be made using a one-sided, two-sample Score test (Farrington & Manning) with a Type I error rate (α) of 0.025. The reference group proportion is assumed to be 0.65. To detect a proportion ratio (P1 / P2) of 1.2 (or P1 of 0.78) with sample sizes of 50 for the treatment group and 50 for the reference group, the power is 0.10144.

Dropout-Inflated Sample Size

	Sample Size			Dropout-Inflated Enrollment Sample Size			Expected Number of Dropouts		
Dropout Rate	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

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.

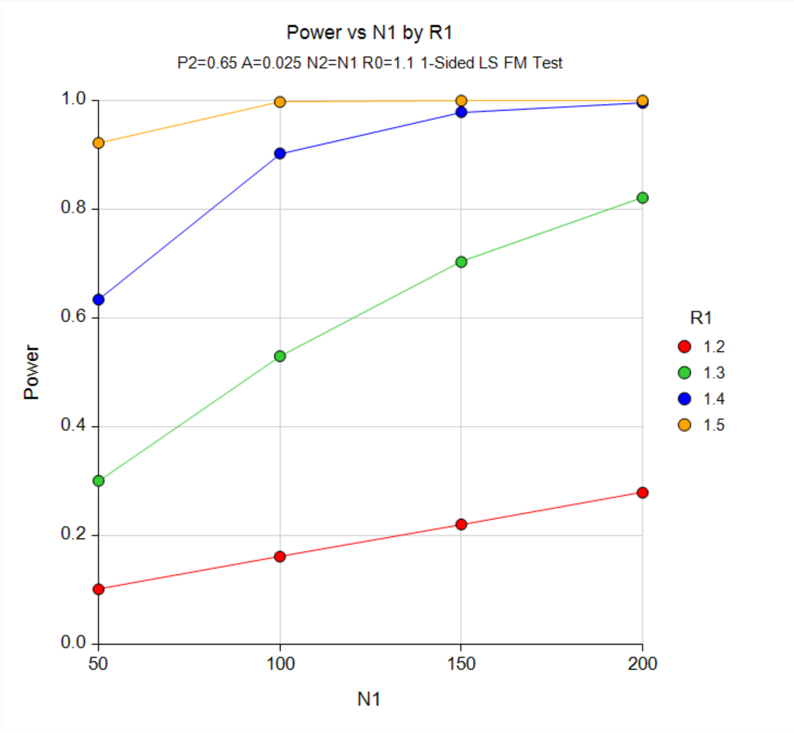
References

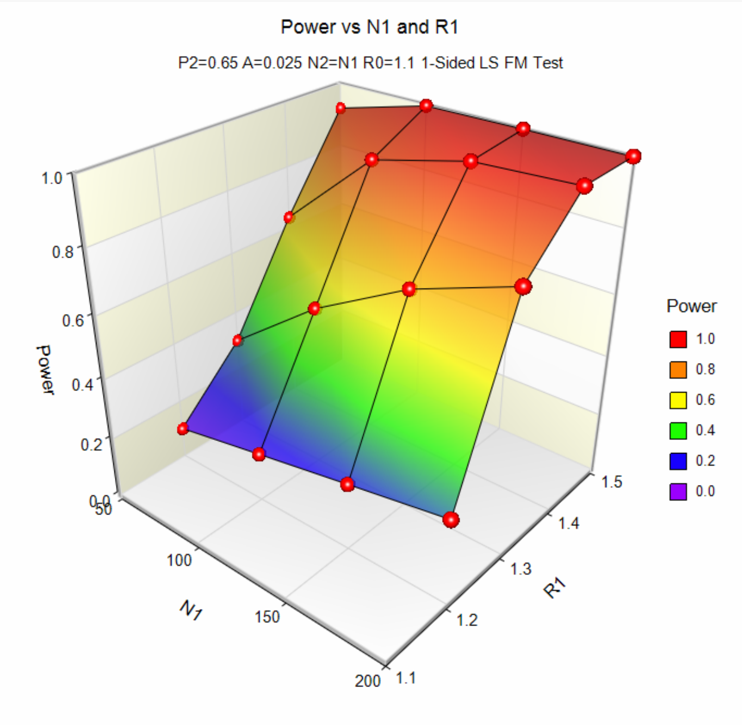
Chow, S.C., Shao, J., and Wang, H. 2008. Sample Size Calculations in Clinical Research, Second Edition. Chapman & Hall/CRC. Boca Raton, Florida.
Farrington, C. P. and Manning, G. 1990. 'Test Statistics and Sample Size Formulae for Comparative Binomial Trials with Null Hypothesis of Non-Zero Risk Difference 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 charts. These charts give us a quick look at the sample size that will be required for various values of R1.

Example 2 – Finding the Sample Size

Continuing with the scenario given in Example 1, the researchers want to determine the sample size necessary for each value of R1 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**
Alternative Hypothesis ....**One-Sided (H1: P1/P2 > R0)**
Test Type..**Likelihood Score (Farr. & Mann.)**
Power..**0.80**
Alpha...**0.025**
Group Allocation ....**Equal (N1 = N2)**
R0 (Ratio|H0 = P1.0/P2) **1.1**
R1 (Ratio|H1 = P1.1/P2) **1.2 1.3 1.4 1.5**
P2 (Group 2 Proportion)................................**0.65**

Output

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

Numeric Results

Solve For: Sample Size
Test Statistic: Farrington & Manning Likelihood Score Test
Hypotheses: H0: P1 / P2 ≤ R0 vs. H1: P1 / P2 > R0

Power		Sample Size			Proportions			Ratio		
Target	Actual*	N1	N2	N	P1|H0 P1.0	P1|H1 P1.1	Reference P2	Ratio|H0 R0	Ratio|H1 R1	Alpha
0.8	0.80013	831	831	1662	0.715	0.780	0.65	1.1	1.2	0.025
0.8	0.80156	190	190	380	0.715	0.845	0.65	1.1	1.3	0.025
0.8	0.80020	74	74	148	0.715	0.910	0.65	1.1	1.4	0.025
0.8	0.80818	35	35	70	0.715	0.975	0.65	1.1	1.5	0.025

* Power was computed using the normal approximation method.

The required sample size will depend a great deal on the value of R1. Any effort spent determining an accurate value for R1 will be worthwhile.

Example 3 – Comparing the Power of the Three Test Statistics

Continuing with Example 2, the researchers want to determine which of the eight 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 800 and 1000 when R1 is 1.2.

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**
Alternative Hypothesis ....**One-Sided (H1: P1/P2 > R0)**
Test Type...**Likelihood Score (Farr. & Mann.)**
Alpha..**0.025**
Group Allocation ....**Equal (N1 = N2)**
Sample Size Per Group **800 900 1000**
R0 (Ratio|H0 = P1.0/P2) **1.1**
R1 (Ratio|H1 = P1.1/P2) **1.2**
P2 (Group 2 Proportion)...**0.65**

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 Three Different Tests

Hypotheses: H0: P1 / P2 ≤ R0 vs. H1: P1 / P2 > R0

Sample Size							Power		
N1	N2	N	P2	R0	R1	Target Alpha	F.M. Score	M.N. Score	G.N. Score
800	800	1600	0.65	1.1	1.2	0.025	0.7855	0.7854	0.7855
900	900	1800	0.65	1.1	1.2	0.025	0.8311	0.8311	0.8305
1000	1000	2000	0.65	1.1	1.2	0.025	0.8678	0.8674	0.8674

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

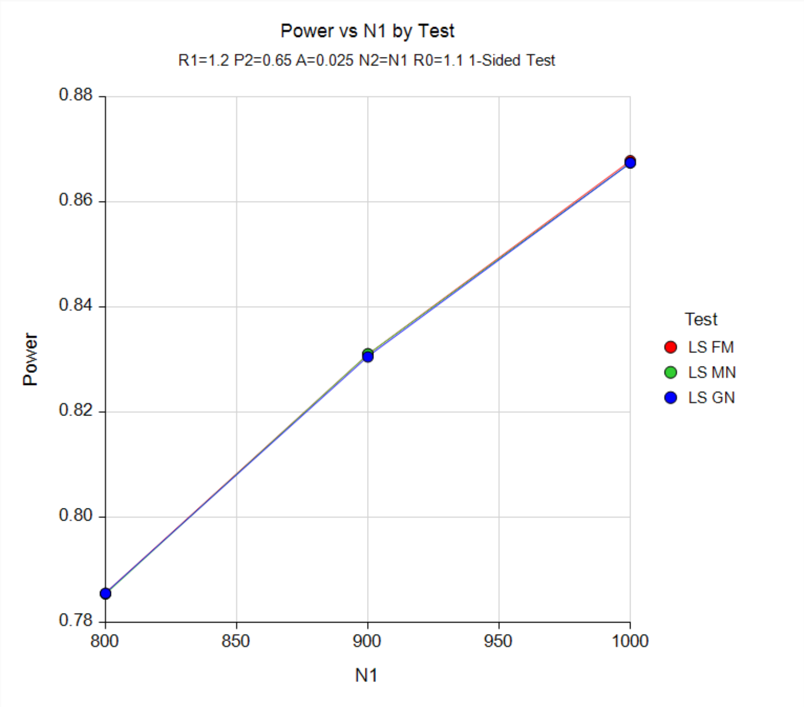
Actual Alpha Comparison of Three Different Tests

Hypotheses: H0: P1 / P2 ≤ R0 vs. H1: P1 / P2 > R0

Sample Size						Alpha			
N1	N2	N	P2	R0	R1	Target	F.M. Score	M.N. Score	G.N. Score
800	800	1600	0.65	1.1	1.2	0.025	0.0250	0.025	0.0250
900	900	1800	0.65	1.1	1.2	0.025	0.0250	0.025	0.0250
1000	1000	2000	0.65	1.1	1.2	0.025	0.0251	0.025	0.0251

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

Plots



All three test statistics have about the same power for all sample sizes studied.

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**
Alternative Hypothesis .... **One-Sided (H1: P1/P2 > R0)**
Test Type .... **Likelihood Score (Farr. & Mann.)**
Alpha.. **0.025**
Group Allocation .... **Equal (N1 = N2)**
Sample Size Per Group **800 900 1000**
R0 (Ratio|H0 = P1.0/P2) **1.1**
R1 (Ratio|H1 = P1.1/P2) **1.2**
P2 (Group 2 Proportion)................................ **0.65**

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: P1 / P2 ≤ R0 vs. H1: P1 / P2 > R0

Sample Size						Normal Approximation		Binomial Enumeration	
N1	N2	N	P2	R0	R1	Power	Alpha	Power	Alpha
800	800	1600	0.65	1.1	1.2	0.78503	0.025	0.78552	0.0250
900	900	1800	0.65	1.1	1.2	0.83049	0.025	0.83109	0.0250
1000	1000	2000	0.65	1.1	1.2	0.86734	0.025	0.86783	0.0251

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

Example 5 – Validation of Power Calculations using Blackwelder (1993)

Blackwelder (1993), page 695, presents a table of power values for several scenarios using the risk ratio. The second line of the table presents the results for the following scenario: P2 = 0.04, R0 = 0.3, R1=0.1, N1=N2=1044, one-sided alpha = 0.05, and beta = 0.20. Using the Farrington and Manning likelihood-score test statistic, he found the binomial enumeration power to be 0.812, the actual alpha to be 0.044, and, using the asymptotic formula, the approximate power to be 0.794.

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 5(a or b)** 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**
Alternative Hypothesis .... **One-Sided (H1: P1/P2 < R0)**
Test Type.. **Likelihood Score (Farr. & Mann.)**
Alpha.. **0.05**
Group Allocation .... **Equal (N1 = N2)**
Sample Size Per Group **1044**
R0 (Ratio|H0 = P1.0/P2) **0.3**
R1 (Ratio|H1 = P1.1/P2) **0.1**
P2 (Group 2 Proportion).. **0.04**

Output

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

Numeric Results

Solve For: Power
Test Statistic: Farrington & Manning Likelihood Score Test
Hypotheses: H0: P1 / P2 ≥ R0 vs. H1: P1 / P2 < R0

	Sample Size			Proportions			Ratio		Alpha	
Power*	**N1**	**N2**	**N**	**P1|H0 P1.0**	**P1|H1 P1.1**	**Reference P2**	**Ratio|H0 R0**	**Ratio|H1 R1**	**Target**	**Actual***
0.81178	1044	1044	2088	0.012	0.004	0.04	0.3	0.1	0.05	0.0444

* Power and actual alpha were computed using binomial enumeration of all possible outcomes.

PASS calculated the power to be 0.81178 and the actual alpha to be 0.0444, which round to Blackwelder's values.

Next, to calculate the asymptotic power, we make the following changes to the template:

Design Tab

Power Calculation Method .... **Normal Approximation**

Numeric Reports

Numeric Results

Solve For: Power
Test Statistic: Farrington & Manning Likelihood Score Test
Hypotheses: H0: P1 / P2 ≥ R0 vs. H1: P1 / P2 < R0

	Sample Size			Proportions			Ratio		
Power*	N1	N2	N	P1|H0 P1.0	P1|H1 P1.1	Reference P2	Ratio|H0 R0	Ratio|H1 R1	Alpha
0.79373	1044	1044	2088	0.012	0.004	0.04	0.3	0.1	0.05

* Power was computed using the normal approximation method.

PASS also calculated the power to be 0.794.