Chapter 253

Tests for Two Ordered Categorical Variables (Legacy)

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 $C_1, \ldots, C_K$. 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

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

This measures the advantage of the experimental group over the control group. A positive θ_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 θ. 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 θ is small and *n* is large. The test statistic and power formulae are as follows:

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

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

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

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

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

$$\mu_Y = \theta V$$

$$\sigma_Y^2 = V$$

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

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,

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

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

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

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

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 θ 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

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**
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**

Output

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

Numeric Reports

Numeric Results for Two-Sample, Ordered-Categorical Test

Solve For: Power
Hypotheses: H0: θ = 0 vs. H1: θ ≠ 0

	Sample Size			Log Odds Ratio of Treatment	Category 1 Proportion		
Power	N1	N2	N	θ	Control PC(1)	Treatment PE(1)	Alpha
0.1726	30	30	60	0.5	0.2	0.2919	0.05
0.5310	30	30	60	1.0	0.2	0.4046	0.05
0.8653	30	30	60	1.5	0.2	0.5284	0.05
0.9828	30	30	60	2.0	0.2	0.6488	0.05
0.2172	40	40	80	0.5	0.2	0.2919	0.05
0.6564	40	40	80	1.0	0.2	0.4046	0.05
0.9444	40	40	80	1.5	0.2	0.5284	0.05

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Power The probability of rejecting a false null hypothesis when the alternative hypothesis is true.
N1 and N2 The number of subjects in the first and second groups.
N The total sample size. N = N1 + N2.
θ The log odds ratio. θ = log[odds(treatment) / odds(control)].
PC(1) The proportion of the first (best) category in the control group.
PE(1) The proportion of the first (best) category in the treatment group.
Alpha 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 0.5 when using a two-sided test with a Type I error rate (α) of 0.05.

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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%	30	30	60	38	38	76	8	8	16
20%	40	40	80	50	50	100	10	10	20
20%	50	50	100	63	63	126	13	13	26
20%	60	60	120	75	75	150	15	15	30
20%	70	70	140	88	88	176	18	18	36
20%	80	80	160	100	100	200	20	20	40
20%	90	90	180	113	113	226	23	23	46
20%	100	100	200	125	125	250	25	25	50
20%	110	110	220	138	138	276	28	28	56
20%	120	120	240	150	150	300	30	30	60
20%	130	130	260	163	163	326	33	33	66
20%	140	140	280	175	175	350	35	35	70
20%	150	150	300	188	188	376	38	38	76

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, 38 subjects should be enrolled in Group 1, and 38 in Group 2, to obtain final group sample sizes of 30 and 30, respectively.

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.

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, N

This is the number of subjects in the control group, experimental group, and both groups (total), respectively.

θ (Log Odds Ratio of Treatment)

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

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

PC(1)

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

PE(1)

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

Alpha

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

Category Probability Distribution

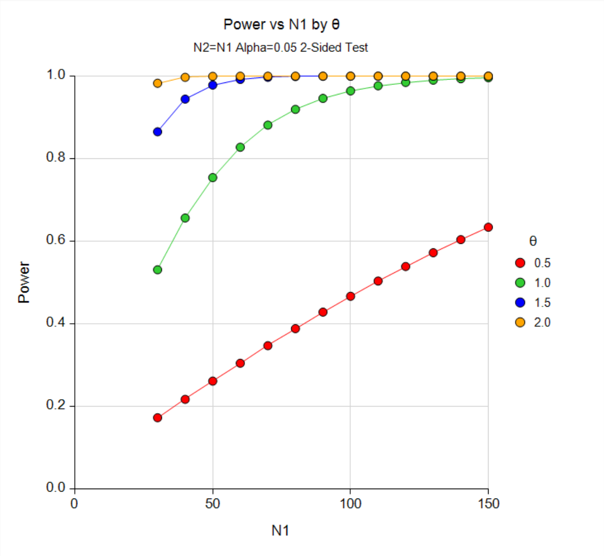
Category Probability Distribution

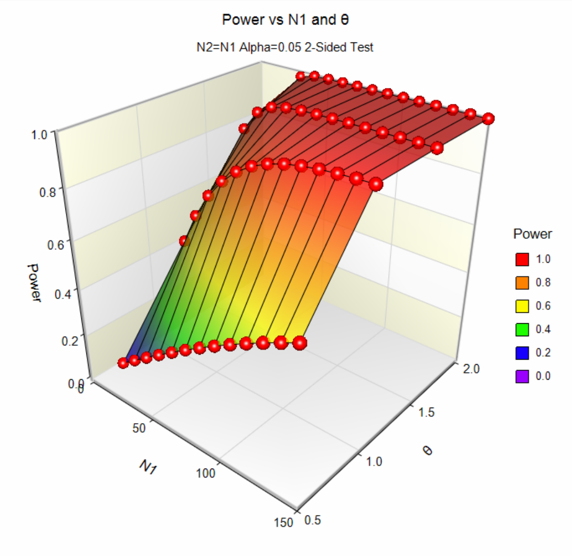
θ	Pr(1)	Pr(2)	Pr(3)	Pr(4)
0.0	0.2000	0.5000	0.2000	0.1000
0.5	0.2919	0.5018	0.1432	0.0631
1.0	0.4046	0.4592	0.0969	0.0393
1.5	0.5284	0.3843	0.0631	0.0242
2.0	0.6488	0.2964	0.0400	0.0148

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

Plots Section

Plots





These plots give 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 θ is 0.9.

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**
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 for Two-Sample, Ordered-Categorical Test

Solve For: Sample Size
Hypotheses: H0: θ = 0 vs. H1: θ ≠ 0

	Sample Size			Log Odds Ratio of Treatment	Category 1 Proportion		
Power	N1	N2	N	θ	Control PC(1)	Treatment PE(1)	Alpha
0.9	92	92	184	0.9	0.2	0.3808	0.05

Category Probability Distribution

θ	Pr(1)	Pr(2)	Pr(3)	Pr(4)
0.0	0.2000	0.5000	0.2000	0.1000
0.9	0.3808	0.4708	0.1052	0.0432

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 θ 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

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 .... **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 for Two-Sample, Ordered-Categorical Test

Solve For: Sample Size
Hypotheses: H0: θ = 0 vs. H1: θ ≠ 0

	Sample Size			Log Odds Ratio of Treatment	Category 1 Proportion		
Power	N1	N2	N	θ	Control PC(1)	Treatment PE(1)	Alpha
0.9	94	94	189	0.887	0.2	0.3777	0.05

Category Probability Distribution

θ	Pr(1)	Pr(2)	Pr(3)	Pr(4)
0.000	0.2000	0.5000	0.2000	0.1000
0.887	0.3777	0.4723	0.1063	0.0438

PASS matches the required sample size of 94 per group.