Chapter 377

Mixed Models Tests for Two Proportions in a 3-Level Hierarchical Design (Level-1 Randomization)

Introduction

This procedure calculates power and sample size for a three-level hierarchical mixed-effects logistic regression model which is randomized at the **first** level. The goal of the study is to compare two group proportions. The study may be cross-sectional or longitudinal.

In a *cross-sectional* version of this design, students (first level units) are nested in classrooms (second level units) which are nested in schools (third level units). Each student is randomized into one of two intervention groups, e.g., treatment and control.

In a *longitudinal* version of this design, repeated measurements (first level units) are nested in patients (second level units) which are nested in clinics (third level units). Each repeated measure is randomized into one of two intervention groups, e.g., treatment and control.

Note that companion procedures analyze the other cases in which the randomization occurs at the second or third level.

Technical Details

Our formulation comes from Ahn, Heo, and Zhang (2015), chapter 6, section 6.7.3, pages 228-230. The hierarchical mixed model used for the analysis is

$$\log\left(\frac{p_{ijk}}{1-p_{ijk}}\right) = \beta_0 + \delta X_{ijk} + u_i + u_{j(i)}$$

where

Y_{ijk}	is the binary response of the k^{th} level-1 unit of the j^{th} level-2 unit of the i^{th} level-3 unit.
p_{ijk}	is an expected value defined by $p_{ijk} = E(Y_{ijk}\|X_{ijk})$. Assume $\left[p_{ijk}\middle\|(X_{ijk}=0)\right] = p_2$ and $\left[p_{ijk}\middle\|(X_{ijk}=1)\right] = p_1$
β_0	is the fixed intercept.
δ	is the treatment effect of interest. It is the difference between the two group proportions.
X_{ijk}	is an indicator variable that is 1 if i^{th} unit is in group 1 and 0 if it is in group 2.
u_i	is the level-3 random intercept effect for the i^{th} level-3 unit. It is distributed as $N(0,\sigma_3^2)$.
$u_{j(i)}$	is the level-2 random intercept effect for the $j(i)^{th}$ level-2 unit. It is distributed as $N(0,\sigma_2^2)$.

ρ_1 is the correlation among level-1 units which are in a particular level-2 unit. For fixed models like this, $\rho_1 = Corr\left(Y_{ijk}, Y_{ijk'}\right) = (\sigma_2^2 + \sigma_3^2)/\sigma^2$.

ρ_2 is the correlation among level-2 units which are in a particular level-3 unit. For fixed models like this, $\rho_2 = Corr\left(Y_{ijk}, Y_{ij'k'}\right) = (\sigma_3^2)/\sigma^2$. However, the power does not depend on this value.

C is the number of level-3 units.

K is the number of level-2 units per level-3 unit.

M_1 is the number of level-1 units per level-2 unit assigned to group 1.

M_2 is the number of level-1 units per level-2 unit assigned to group 2.

The test of significance of the X_{ijk} term in the logistic model is the test statistic of interest. It tests the difference of the two group proportions.

Assume that $\delta = p_1 - p_2$ is to be tested using a z-test (large sample). The statistical hypotheses are $H_0: \delta = 0$ vs. $H_a: \delta \neq 0$. The test statistic is the regression coefficient of the X_{ijk} term in a mixed model.

The power can be calculated using

$$Power = \Phi\left\{\frac{|p_1 - p_2|\sqrt{M_2CK/f_1} - \Phi^{-1}(1-\alpha/2)\sqrt{(1+1/\lambda)\bar{p}(1-\bar{p})}}{\sqrt{p_2(1-p_2) + p_1(1-p_1)/\lambda}}\right\}$$

where $\lambda = M_1/M_2$, $\bar{p} = (M_1p_1 + M_2p_2)/(M_1 + M_2)$, and $f_1 = 1 - \rho_1$.

Example 1 – Calculating Power

Suppose that a three-level hierarchical design is planned in which there will be students (level-1) which are nested in classrooms (level-2) which are nested in schools (level-3). This analysis will calculate the power for testing the significance of the difference in proportions of two interventions. There will be one measurement per student and treatments will be applied to students (level-1 units).

The analysis will use a mixed logistic regression model. The following parameter settings are to be used for the power analysis: $P1$ = 0.6; $P2$ = 0.5; $\rho 1$ = 0.02; C = 6, 8, 10; $M1$ = 5, 10, 15, 20; $M2$ = $M1$; and α = 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**
Alpha... **0.05**
C (Level 3 Units) **5**
K (Level 2 Units Per Level 3 Unit)................. **6 8 10**
M1 (Level 1 Units Assigned to Group 1) **5 10 15 20**
M2 (Level 1 Units Assigned to Group 2) **M1**
P1 Input Type .... **Proportions**
P1 (Group 1 Proportion|H1) **0.6**
P2 (Group 2 Proportion)................................ **0.5**
ρ1 (Correlation Among Level 1 Units)........... **0.02**

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 = Control
Hypotheses: H0: P1 = P2 vs. H1: P1 ≠ P2

				Number of Level 1 Units per Level 2 Unit		Proportion				
Power	Total Sample Size N	Number of Level 3 Units C	Number of Level 2 Units per Level 3 Unit K	Group 1 M1	Group 2 M2	Group 1 P1	Group 2 P2	Difference P1 - P2	Correlation Among Level 1 Units ρ1	Alpha
0.4197	300	5	6	5	5	0.6	0.5	0.1	0.02	0.05
0.7018	600	5	6	10	10	0.6	0.5	0.1	0.02	0.05
0.8624	900	5	6	15	15	0.6	0.5	0.1	0.02	0.05
0.9412	1200	5	6	20	20	0.6	0.5	0.1	0.02	0.05
0.5283	400	5	8	5	5	0.6	0.5	0.1	0.02	0.05
0.8202	800	5	8	10	10	0.6	0.5	0.1	0.02	0.05
0.9412	1200	5	8	15	15	0.6	0.5	0.1	0.02	0.05
0.9826	1600	5	8	20	20	0.6	0.5	0.1	0.02	0.05
0.6224	500	5	10	5	5	0.6	0.5	0.1	0.02	0.05
0.8956	1000	5	10	10	10	0.6	0.5	0.1	0.02	0.05
0.9763	1500	5	10	15	15	0.6	0.5	0.1	0.02	0.05
0.9952	2000	5	10	20	20	0.6	0.5	0.1	0.02	0.05

Power The probability of rejecting a false null hypothesis when the alternative hypothesis is true.
N The total number of Level-1 units.
C The number of Level-3 units.
K The average number of Level-2 units per Level-3 unit.
M1 and M2 The average number of Level-1 units per Level-2 unit assigned to group 1 and 2, respectively.
P1 The proportion for group 1 (treatment group) assuming the alternative hypothesis.
P2 The proportion for group 2 (control group). This is the proportion in the standard, reference, baseline, or control group.
P1 - P2 The difference in the group proportions assumed by the alternative hypothesis.
ρ1 The correlation among Level-1 units in a particular Level-2 unit.
Alpha The probability of rejecting a true null hypothesis.

Summary Statements

A 2-group 3-level hierarchical design will have level-1 units (e.g., students, subjects, or patients) in level-2 units (e.g., classes, clinics, or hospitals) in level-3 units (e.g., schools, regions, or networks) with random assignment of level-1 units to each of the 2 groups (level-1 randomization). This design will be used to test the difference between two proportions, using the appropriate term of the hierarchical mixed-effects logistic regression model, with a Type I error rate (α) of 0.05. The correlation of level-1 units within a level-2 unit is assumed to be 0.02. To detect a proportion difference (P1 - P2) of 0.1 (with P1 = 0.6 and P2 = 0.5), with 5 level-3 units, with 6 level-2 units in each level-3 unit, and within each level-2 unit, 5 level-1 units in Group 1 and 5 level-1 units in Group 2 (for a grand total of 300 level-1 units), the power is 0.4197.

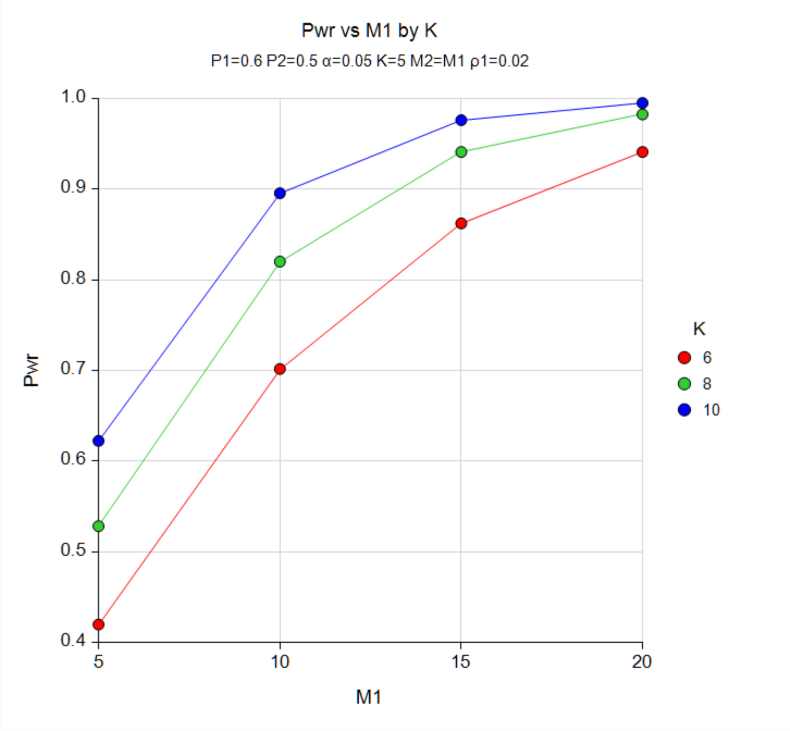
References

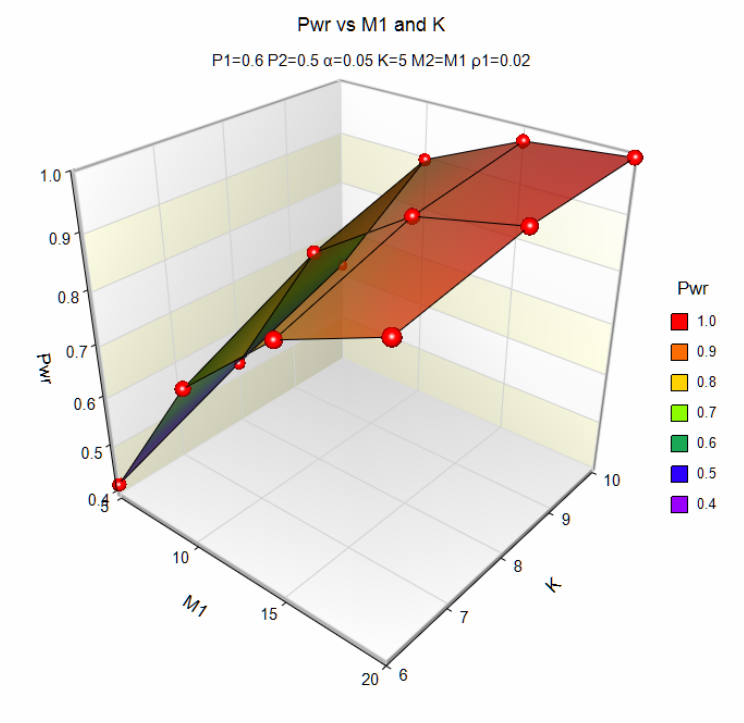
Ahn, C., Heo, M., and Zhang, S. 2015. Sample Size Calculations for Clustered and Longitudinal Outcomes in Clinical Research. CRC Press. New York.

This report shows the power for each of the scenarios.

Plots Section

Plots





These plots show the power versus the level-1 count for the three values of K.

Example 2 – Calculating Sample Size (Number Level 1 Units Per Level 2 Unit)

Continuing with the last example, suppose the researchers want to determine the number of level 2 needed to achieve 90% power for the three values of K.

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 .... **M1 (Number Level 1 Units Per Level 2 Unit)**
Power... **0.90**
Alpha.. **0.05**
C (Level 3 Units) **5**
K (Level 2 Unit Per Level 3 Unit).................... **6 8 10**
M2 (Level 1 Units Assigned to Group 2) **M1**
P1 Input Type .... **Proportions**
P1 (Group 1 Proportion|H1) **0.6**
P2 (Group 2 Proportion)................................ **0.5**
ρ1 (Correlation Among Level 1 Units)........... **0.02**

Output

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

Numeric Results

Solve For: M1 (Number Level 1 Units Per Level 2 Unit)
Groups: 1 = Treatment, 2 = Control
Hypotheses: H0: P1 = P2 vs. H1: P1 ≠ P2

	Total	Number of	Number of	Number of Level 1 Units per Level 2 Unit		Proportion			Correlation	
Power	Sample Size N	Level 3 Units C	Level 2 Units per Level 3 Unit K	Group 1 M1	Group 2 M2	Group 1 P1	Group 2 P2	Difference P1 - P2	Among Level 1 Units ρ1	Alpha
0.9013	1020	5	6	17	17	0.6	0.5	0.1	0.02	0.05
0.9067	1040	5	8	13	13	0.6	0.5	0.1	0.02	0.05
0.9214	1100	5	10	11	11	0.6	0.5	0.1	0.02	0.05

This report shows the sample size for each of the scenarios.

Example 3 – Validation using Ahn, Heo, and Zhang (2015)

Ahn, Heo, and Zhang (2015) page 231 provide a table in which several scenarios are reported. We will validate this procedure by the first row of the table. The following parameter settings were for the analysis: power = 0.80; *P1* = 0.5; *P2* = 0.4; ρ1 = 0.1; *C* = 20; *K* = 4; and α = 0.05. These settings resulted in a value of *M1* and *M2* (their $N_1^{(0)}$) of 5 and an attained power of 0.851.

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 .... **M1 (Number Level 1 Units Per Level 2 Unit)**
Power... **0.80**
Alpha.. **0.05**
C (Level 3 Units) **24**
K (Level 2 Units Per Level 3 Unit).................. **4**
M (Level 1 Units Per Level 2 Unit) **5**
P1 Input Type .... **Proportions**
P1 (Group 1 Proportion|H1) **0.5**
P2 (Group 2 Proportion)................................. **0.4**
ρ1 (Correlation Among Level 1 Units)............ **0.1**
ρ2 (Correlation Among Level 2 Units)............ **0.05**

Output

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

Numeric Results

Solve For: M1 (Number Level 1 Units Per Level 2 Unit)
Groups: 1 = Treatment, 2 = Control
Hypotheses: H0: P1 = P2 vs. H1: P1 ≠ P2

				Number of Level 1 Units per Level 2 Unit		Proportion				
Power	Total Sample Size N	Number of Level 3 Units C	Number of Level 2 Units per Level 3 Unit K	Group 1 M1	Group 2 M2	Group 1 P1	Group 2 P2	Difference P1 - P2	Correlation Among Level 1 Units ρ1	Alpha
0.8512	800	20	4	5	5	0.5	0.4	0.1	0.1	0.05

PASS calculates the same values of *M1* and power: 5 and 0.8512.