Chapter 378

Mixed Models Tests for Two Proportions in a 3-Level Hierarchical Design (Level-2 Randomization)

Introduction

This procedure calculates power and sample size for a three-level hierarchical mixed-effects logistic regression model which is randomized at the **second** 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 classroom 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 patient 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 first, or third, level.

Technical Details

Our formulation comes from Ahn, Heo, and Zhang (2015), chapter 6, section 6.7.2, pages 225-228. 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 $p_{ijk} = E(Y_{ijk} \vert X_{ijk})$. Assume $\left[p_{ijk} \middle\vert (X_{ijk} = 0)\right] = p_2$ and $\left[p_{ijk} \middle\vert (X_{ijk} = 1)\right] = p_1$
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 $j(i)^{\text{th}}$ 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, $\rho_2 = Corr\left(Y_{ijk}, Y_{ij'k'}\right) = (\sigma_3^2)/\sigma^2$.

C is the number of level-3 units.

K_1 is the number of level-2 units assigned to group 1.

K_2 is the number of level-2 units assigned to group 2.

M is the number of level-1 units per level-2 unit.

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 $H_0\colon \delta = 0$ vs. $H_a\colon \delta \neq 0$. The test statistic is the regression coefficient of the X_{ijk} term in a mixed model.

The power can be calculated using

$$Power = \Phi\left\{\frac{|p_1 - p_2|\sqrt{K_2 CM/f_2} - \Phi^{-1}(1-\alpha/2)\sqrt{(1+1/\lambda)\bar{p}(1-\bar{p})}}{\sqrt{p_2(1-p_2) + p_1(1-p_1)/\lambda}}\right\}$$

where $\lambda = K_1/K_2$, $\bar{p} = (K_1 p_1 + K_2 p_2)/(K_1 + K_2)$, and $f_2 = 1 + (M-1)\rho_1 - M\rho_2$.

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 classrooms (level-2 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; $P2 = 0.5$; $\rho 1 = 0.02$; $\rho 2 = 0.01$; $C = 6$; $M = 5, 10$; $\alpha = 0.05$; and $K1 = K2$ = 5 to 20 by 5.

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)	**6**
K1 (Level 2 Unit Assigned to Group 1)	**5 10 15 20**
K2 (Level 2 Unit Assigned to Group 2)	**K1**
M (Level 1 Units Per Level 2 Unit)	**5 10**
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**
ρ2 (Correlation Among Level 2 Units)	**0.01**

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 2 Units per Level 3 Unit				Proportion			Correlation		
Power	Total Sample Size N	Number of Level 3 Units C	Group 1 K1	Group 2 K2	Total K	Number of Level 1 Units per Level 2 Unit M	Group 1 P1	Group 2 P2	Difference P1 - P2	Level 1 Units ρ1	Level 2 Units ρ2	Alpha
0.4029	300	6	5	5	10	5	0.6	0.5	0.1	0.02	0.01	0.05
0.6595	600	6	5	5	10	10	0.6	0.5	0.1	0.02	0.01	0.05
0.6802	600	6	10	10	20	5	0.6	0.5	0.1	0.02	0.01	0.05
0.9188	1200	6	10	10	20	10	0.6	0.5	0.1	0.02	0.01	0.05
0.8452	900	6	15	15	30	5	0.6	0.5	0.1	0.02	0.01	0.05
0.9844	1800	6	15	15	30	10	0.6	0.5	0.1	0.02	0.01	0.05
0.9303	1200	6	20	20	40	5	0.6	0.5	0.1	0.02	0.01	0.05
0.9974	2400	6	20	20	40	10	0.6	0.5	0.1	0.02	0.01	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.
K1, K2, and K	The average number of Level-2 units per Level-3 unit assigned to groups 1, 2, and both, respectively.
M	The average number of Level-1 units per Level-2 unit.
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.
ρ2	The correlation among Level-2 units in a particular Level-3 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-2 units to each of the 2 groups (level-2 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, and the correlation of level-2 units within a level-3 unit is assumed to be 0.01. To detect a proportion difference (P1 - P2) of 0.1 (with P1 = 0.6 and P2 = 0.5), with 6 level-3 units, and within each level-3 unit, 5 level-2 units in Group 1 and 5 level-2 units in Group 2, with 5 level-1 units in each level-2 unit (for a grand total of 300 level-1 units), the power is 0.4029.

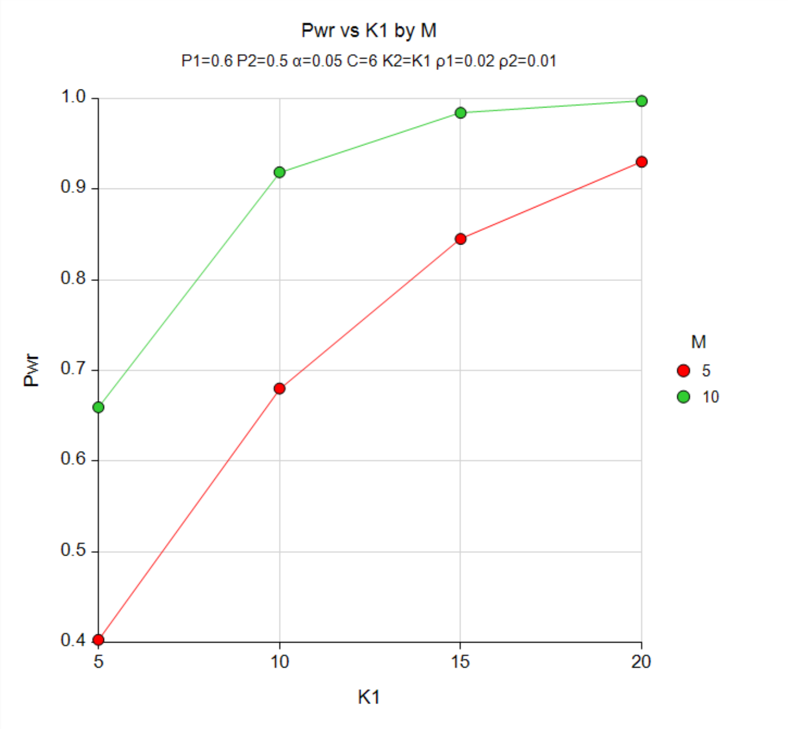
References

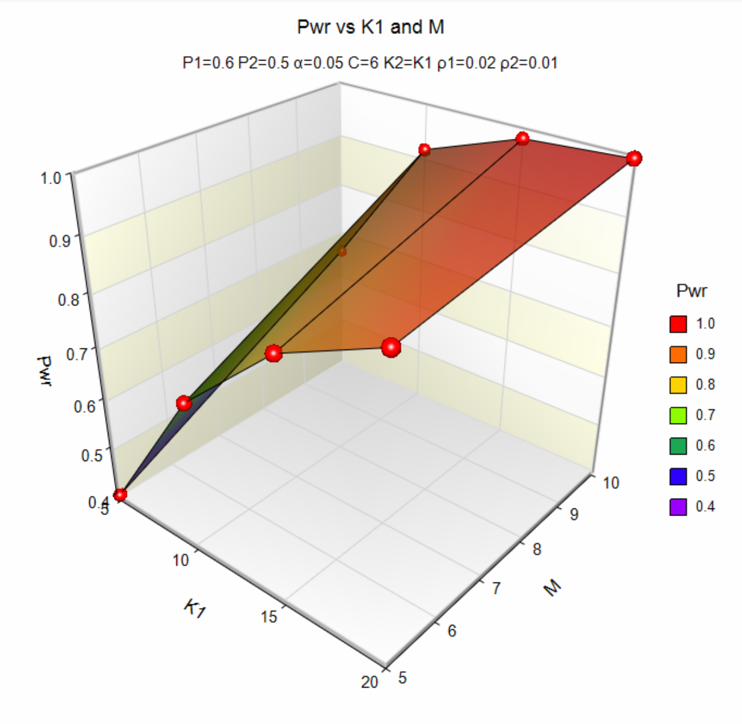
Ahn, C., Heo, M., and Zhang, S. 2015. Sample Size Calculations for Clustered and Longitudinal Outcomes in Clinical Research. CRC Press. New York.

This report shows the power for each of the scenarios.

Plots Section

Plots





This plot shows the power versus the level-2 count for the two values of M.

Example 2 – Calculating Sample Size (Number of Level 2 Units per Level 3 Unit)

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

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 .... **K1 (Number Level 2 Units Per Level 3 Unit)**
Power.. **0.90**
Alpha.. **0.05**
C (Level 3 Units) **6**
K2 (Level 2 Unit Assigned to Group 2)........... **K1**
M (Level 1 Units Per Level 2 Unit) **5 10**
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**
ρ2 (Correlation Among Level 2 Units)........... **0.01**

Output

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

Numeric Results

Solve For: K1 (Number Level 2 Units Per Level 3 Unit)
Groups: 1 = Treatment, 2 = Control
Hypotheses: H0: P1 = P2 vs. H1: P1 ≠ P2

Power	Total Sample Size N	Number of Level 3 Units C	Number of Level 2 Units per Level 3 Unit: Group 1 K1	Group 2 K2	Total K	Number of Level 1 Units per Level 2 Unit M	Proportion: Group 1 P1	Group 2 P2	Difference P1 - P2	Correlation: Level 1 Units ρ1	Level 2 Units ρ2	Alpha
0.9034	1080	6	18	18	36	5	0.6	0.5	0.1	0.02	0.01	0.05
0.9188	1200	6	10	10	20	10	0.6	0.5	0.1	0.02	0.01	0.05

This report shows the power for each of the scenarios.

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

Ahn, Heo, and Zhang (2015) page 228 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; ρ*1 = *0.1; ρ*2 = *0.05; C = 24; M = 5;* and *α = 0.05*. These settings resulted in a value of *K1* and *K2* (their $N_2^{(0)}$) of 4 and an attained power of 0.829.

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	**K1 (Number Level 2 Units Per Level 3 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: K1 (Number Level 2 Units Per Level 3 Unit)
Groups: 1 = Treatment, 2 = Control
Hypotheses: H0: P1 = P2 vs. H1: P1 ≠ P2

			Number of Level 2 Units per Level 3 Unit				Proportion			Correlation		
Power	Total Sample Size N	Number of Level 3 Units C	Group 1 K1	Group 2 K2	Total K	Number of Level 1 Units per Level 2 Unit M	Group 1 P1	Group 2 P2	Difference P1 - P2	Level 1 Units ρ1	Level 2 Units ρ2	Alpha
0.8286	960	24	4	4	8	5	0.5	0.4	0.1	0.1	0.05	0.05

PASS calculates the same values of *K1* and power: 4 and 0.8286.