# Contents

1	Sam	ple Size and Power	<b>2</b>
	1.1	Short review	2
	1.2	Sample Size for Comparing Two Proportions	2

## 1 Sample Size and Power

The goal for most studies employing logistic regression is to test whether different predictors have an effect on a binary response variable.

We want to discuss the required sample size for detecting an effect of a given size.

#### 1.1 Short review

When planning a study for estimating a success probability  $\pi$  with a  $(1 - \alpha) \times 100\%$  confidence interval within a margin of error of M, then the required sample size is

$$n \ge \pi (1 - \pi) \left(\frac{z^*}{M}\right)^2$$

This is based on the large sample Z-confidence interval

$$\hat{\pi} \pm z^* \sqrt{\frac{\pi(1-\pi)}{n}}$$

with Margin of Error of  $z^* \sqrt{\frac{\pi(1-\pi)}{n}}$ .

Then we choose the sample size so that we can be certain the Margin of Error will not exceed M, resulting in the formula for the sample size given above.

### **1.2** Sample Size for Comparing Two Proportions

Most approaches for determining the sample size are based on a different approach.

We ask: What is the required sample size to detect an effect of a certain size using a statistical test at significance level of  $\alpha$ , and and error probability for the error of type 2 of at most  $\beta$  (or power of  $1 - \beta$ )?

For two proportions we would specify,

- How large is the effect we want to be able to detect:  $M = \pi_1 \pi_2$
- The acceptable error probability for an error of type 1:  $\alpha$
- The required power of the test if  $\pi_1 \pi_2 = M$ :  $1 \beta$

These choices mean: If  $|\pi_1 - \pi_2| > M$ , then the probability for not rejecting  $H_0: \pi_1 - \pi_2 = 0$  at significance level of  $\alpha$  should not exceed  $\beta$ .

That is: when using a test at significance level of  $\alpha$ , an error of type II shall be unlikely ( $\beta$ ) if  $H_0$  is violated by at least M.



Using equal sample sizes for the two samples requires approximately:

$$n_1 = n_2 = \frac{(z_{\alpha/2} + z_\beta)^2 (\pi_1 (1 - \pi_1) + \pi_2 (1 - \pi_2))}{(\pi_1 - \pi_2)^2}$$

Note that we do not just need to know M but good estimates for  $\pi_1$  and  $\pi_2$ .

The test for  $H_0: \pi_1 = \pi_2$  is equivalent to the test if the slope in a logistic regression model is zero, when the predictor is binary. Thus this is relevant for finding sample sizes in logistic regression.

#### Example 1

Assume we want to test if the probability for remission is the same for treatment A and treatment B. Former studies have shown that for treatment A 75% of patients achieve remission and we hope for treatment B to improve this rate by at least 5%.

We will use a test at significance level of 5% and require a power of at least 80% if this rate of improvement is correct.

The required sample size is at least

$$n_1 = n_2 = \frac{(1.96 + 0.84)^2 (0.75(0.25) + 0.80(0.2))}{(0.75 - 0.80)^2} = 1089.76$$

Thus we would need at least 1090 observations for each treatment. (Reason: 5% improvement is not that large).

Based on the same idea, when considering the logistic regression model

$$\operatorname{logit}(\pi) = \alpha + \beta_1 x$$

and intends to test  $H_0$ :  $\beta_1 = 0$ , the sample size depends on the distribution of the values for the predictor, x. One needs to be able to guess the probability of success,  $\bar{\pi}$ , when  $x = \bar{x}$ .

The effect size is the odds ratio  $\vartheta$  comparing  $\bar{\pi}$  to the probability of success one standard deviation above the mean,  $\bar{\pi}$ .

Let  $\lambda = \log(\vartheta)$ . an approximate sample size formula for a one sided test is then

$$n = [z_{\alpha} + z_{\beta} \exp(-\lambda^2/4)]^2 (1 + 2\bar{\pi}\delta)/(\bar{\pi}\lambda^2)$$

with

$$\delta = [1 + (1 + \lambda^2) \exp(5\lambda^2/4)] / [1 + \exp(-\lambda^2/4)].$$

The usefulness of this approach and formula is limited, since one usually would not know the mean of the predictor x and  $\bar{\pi}$ .

Using a similar approach to multiple logistic regression would require even more information before one even collects data, and the outcome becomes even more guesswork.