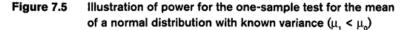
February 11, 2014

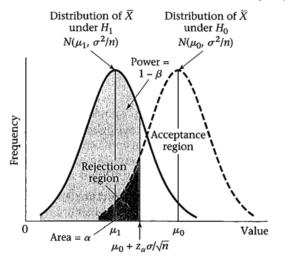
### Power of a test

1. Assuming standard deviation is known. Calculate power based on one-sample z test. A new drug is proposed for people with high intraocular pressure (IOP), to prevent the development of glaucoma. A pilot study is conducted with the drug among 10 patients. Their mean IOP decreases by 5 mm Hg with a sd of 10 mm Hg after 1 month of using the drug. The investigator propose to study 100 participants in the main study. Is this a sufficient sample size for the study?  $H_0: \mu = \mu_0$  vs.  $H_1: \mu < \mu_0$  When the distribution is normal and variance is known.

 $H_0$  is rejected if  $Z < Z_{\alpha}$  and  $H_0$  is accepted otherwise. The best test does not depend on the alternative mean  $\mu_1$  What is the difference?

Power = 1 - P(type II error) = P(reject  $H_0 \mid H_0$  false) =  $P(\frac{\bar{X}-\mu_0}{\sigma/\sqrt{n}} < z_\alpha \mid \mu = \mu_1)$ Under  $H_1, \bar{X} \sim N(\mu_1, \sigma^2/n)$ . Hence, Power =  $P(\frac{\bar{X}-\mu_1}{\sigma/\sqrt{n}} < (\mu_0 + Z_\alpha \sigma/\sqrt{n} - \mu_1)/(\sigma/\sqrt{n})) = P(Z < Z_\alpha + \frac{(\mu_0 - \mu_1)\sigma}{\sqrt{n}}).$ 





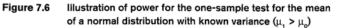
2. Compute the power of the test for the birthweight data with an alternative mean of 115 oz and  $\alpha = 0.05$ , assuming the true standard deviation = 24 oz. We have

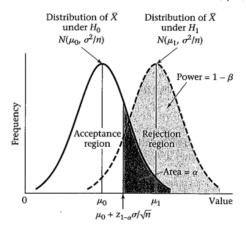
 $\begin{aligned} \mu_0 &= 120oz, \ \mu_1 = 115oz, \ \alpha = 0.05, \ \sigma = 24, n = 100. \\ \text{Power} &= P(Z < Z_\alpha + \frac{(\mu_0 - \mu_1)\sqrt{n}}{\sigma}) \\ P(Z < -1.645 + 5(10)/24) = P(Z < 0.438) = 0.669. \end{aligned}$ 

## Alternative is greater than Null

1. The best test is  $H_0$  rejected if  $Z = \frac{\bar{X} - \mu_0}{\sigma/\sqrt{n}} > Z_{1-\alpha}$  and  $H_0$  is accepted if  $Z \leq Z_{1-\alpha}$ . The power of the test is given by

$$P(\bar{X} > \mu_0 + Z_{1-\alpha}\sigma/\sqrt{n} \mid \mu = \mu_1) = 1 - P(Z < Z_{1-\alpha} + \frac{(\mu_0 - \mu_1)\sqrt{n}}{\sigma})$$
$$= P(Z < Z_\alpha + \frac{(\mu_1 - \mu_0)\sqrt{n}}{\sigma})$$





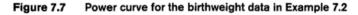
2. Using a 5% level of significance and a sample of size 10, compute the power of the test for the cholesterol data with an alternative mean of 190 mg/dL, a null mean of 175 mg/dL, and a standard deviation of 50 mg/dL. We have  $\mu_0 = 175$ ,  $\mu_1 = 190$ ,  $\alpha = 0.05$ ,  $\sigma = 50$ , n = 10.

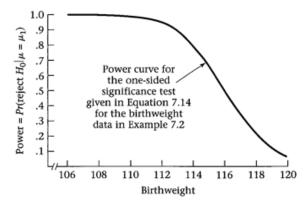
Power = 
$$P(Z < Z_{\alpha} + \frac{(\mu_0 - \mu_1)\sqrt{n}}{\sigma})$$
  
=  $P(Z < -1.645 + 15\sqrt{10}/50)) = P(Z < -0.696) = 0.243$ 

3. Hence power of a one sample Z test for the mean of a normal distribution with known variance (one-sided alternative) for the hypothesis

$$P(Z < Z_{\alpha} + \frac{(|\mu_0 - \mu_1|)\sqrt{n}}{\sigma})$$

- 4. Factors affecting the power:
  - (a) If the significance level is made small ( $\alpha$  decreases),  $Z_{\alpha}$  decreases and hence the power decreases.
  - (b) If the alternative mean is shifted further away from the null mean  $(|\mu_0 \mu_1|$  increases), then the power increases.
  - (c) If the standard deviation of the distribution of individual observation increases ( $\sigma$  increases), then the power decreases.
  - (d) If the sample size increases, then the power increases.
- 5. Power curve for the birthweight data:





6. Two sided alternative: Reject if  $\frac{(\bar{X}-\mu_0}{\sigma/\sqrt{n}} > Z_{1-\alpha/2}$  or  $\frac{(\bar{X}-\mu_0}{\sigma/\sqrt{n}} < Z_{\alpha/2}$ . Hence power

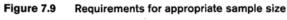
$$\Phi(Z_{\alpha/2} + \frac{(\mu_0 - \mu_1)\sqrt{n}}{\sigma}) + \Phi(Z_{\alpha/2} + \frac{(\mu_1 - \mu_0)\sqrt{n}}{\sigma})$$

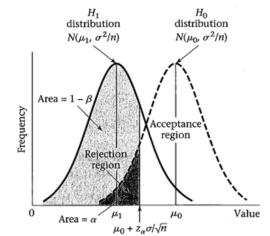
and is approximated by

$$\Phi(Z_{\alpha/2} + \frac{|\mu_0 - \mu_1|\sqrt{n}}{\sigma})$$

# 1 Sample size determination

1. Sample size is important for a study design. Significance level is normally specified. If alternative hypothesis is true, what is the probability of rejecting null? This is the power of the test, typical values are 80% or above. Given a significance level  $\alpha$ , and that the true alternative mean is expected to be  $\mu_1$ , what sample size is needed to be able to detect a significance difference with probability  $1 - \beta$ ?





2. For one sided alternative: Power =  $P(Z < Z_{\alpha} + \frac{(|\mu_0 - \mu_1|)\sqrt{n}}{\sigma}) = 1 - \beta$ . Solve *n* in terms of  $\alpha, \beta, |\mu_0 - \mu_1|$  and  $\sigma$ .

$$n = \frac{(Z_{1-\beta} + Z_{1-\alpha})^2 \sigma^2}{(\mu_0 - \mu_1)^2}$$

- 3. Consider the birthweight data. Suppose that  $\mu_0 = 120$  oz,  $\mu_1 = 115oz$ ,  $\alpha = 0.05$ ,  $\sigma = 24, 1-\beta = .80$ , and we use a one-sided test. C ompute the appropriate sample size needed to conduct the test.  $n = [242(Z_{0.8} + Z_{0.95})^2]/25 = 23.04(.84 + 1.645)^2 = 142.3$
- 4. For 2 sided alternative:

$$n = \frac{(Z_{1-\beta} + Z_{1-\alpha/2})^2 \sigma^2}{(\mu_0 - \mu_1)^2}$$

Factors affecting the sample size:

- (a) The sample size increases as  $\sigma^2$  increases
- (b) The sample size increases as the significance level is made smaller ( $\alpha$  decreases)
- (c) The sample size increases as the required power increases  $(1 \beta \text{ increases})$
- (d) The sample size decreases as the absolute value of the distance between the null and the alternative means  $|\mu_0 \mu_1|$  increases.
- 5. Consider a study of the effect of a calcium-channel-blocking agent on heart rate for patients with unstable angina. Suppose we want at least 80% power for detecting a significant difference if the effect of the drug is to change mean heart rate by 5 beats per minute over 48 hours in either direction and  $\sigma = 10$  beats per minute. How many patients should be enrolled in such a study?

We assume  $\alpha = .05$  and  $\sigma = 10$  beats. We use a two-sided test.  $n = \frac{(Z_{1-\beta}+Z_{1-\alpha/2})^2 \sigma^2}{(\mu_0-\mu_1)^2} = [100(Z_{0.8}+z_{0.975})^2]/25 = 31.36$ . If we know the direction of the effect of the drug,  $n = \frac{(Z_{1-\beta}+Z_{1-\alpha/2})^2 \sigma^2}{(\mu_0-\mu_1)^2} = [100(Z_{0.8}+z_{0.95})^2]/25 = 24.7.$ 

### Sample size calculation based on confidence interval width

We may want to estimate the effect with a given degree of precision. Suppose it is well known that propranolol lowers heart rate over 48 hours when given to patients with angina at standard dosage levels. A new study is proposed using a higher dose of propranolol than the standard one. Investigators are interested in estimating the drop in heart rate with high precision.

The  $100(1-\alpha)\%$  CI for  $\mu$  = true decline in the heart rate is  $\bar{x} \pm t_{n-1,1-\alpha/2}s/\sqrt{n}$ . The width of this CI is  $2t_{n-1,1-\alpha/2}S/\sqrt{n}$ . If we want the interval < L

$$2t_{n-1,1-\alpha/2}s/\sqrt{n} = L, \text{ or } n = 4t_{n-1,1-\alpha/2}^2S^2/L^2$$

### Relationship between hypothesis testing and confidence intervals

Suppose we are testing  $H_0: \mu = \mu_0$  versus  $H_1: \mu \neq \mu_0$ .  $H_0$  is rejected with a two-sided level  $\alpha$  test if and only if the two-sided  $100(1 - \alpha)\%$  confidence interval for  $\mu$  does not contain  $\mu_0$ .  $H_0$  is accepted with two-sided level  $\alpha$  test if and only if the two-sided  $100(1 - \alpha)\%$  confidence interval for  $\mu$  does contain  $\mu_0$ . subsection\*One sample  $\chi^2$  test for the variance or a normal distribution

1. An arteriosonde machine "prints" blood-pressure readings on a tape so that the measurement can be read rather than heard.

- 2. An argument for using such a machine is that the variability of measurement obtained by different observers on the same person will be lower than with a standard bloodpressure cuff.
- 3. Suppose we know from previously published work that  $\sigma^2 = 35$  for  $d_i$  obtained from the standard cuff. We want to test the hypothesis

$$H_0: \sigma^2 = \sigma_0^2 = 35 \, vs. \, \sigma^2 \neq \sigma_0^2$$

How should we perform this test?

4.

$$\begin{split} X^2 &= \frac{(n-1)S^2}{\sigma_0^2} \sim \chi^2_{n-1} \\ P(X^2 < \chi^2_{n-1,\alpha/2}) &= \alpha/2 = P(X^2 > \chi^2_{n-1,1-\alpha/2}) \end{split}$$

5. If  $X^2 < \chi^2_{n-1,\alpha/2}$  or  $X^2 > \chi^2_{n-1,1-\alpha/2}$ , then  $H_0$  is rejected. 6.  $\chi^2_{n-1,\alpha/2} < X^2 < \chi^2_{n-1,1-\alpha/2}$ , then  $H_0$  is accepted.

