角度

- from ci （单样本多用）

    ➢ Smaller sample size -> wider CIs

    ➢ Higher confidence -> wider CIs

    ➢ Larger variability -> wider CIs

    ？？
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- from hypothesis testing

from ci

▶ In studies concerned with detecting an effect

  ▶ If an effect deemed to be clinically or biologically important exists, then there is a high chance of it being detected, i.e. that the analysis will be statistically significant.

  ▶ If the sample is too small, then even if large differences are observed, it will be impossible to show that these are due to anything more than sampling variation.

- 对 **MOE**有要求←**容许误差**

1.3 Example 1 - estimating a single mean

估计单个平均值

An investigator wants to estimate the mean systolic blood pressure in children with congenital heart disease. How many children should be enrolled in the study? The investigator plans on using a 95% confidence interval (so $Z_{\alpha/2}$ =1.96) and wants a **margin of error (MOE) of 5 units**.

The standard deviation of systolic blood pressure is unknown, but the investigators conduct a literature search and find that the standard deviation of systolic blood pressures in children with other cardiac defects is between 15 and 20.

To estimate the sample size, we consider the larger standard deviation in order to obtain the most conservative (largest) sample size.

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1.3 Example 1 - estimating a single mean

- CI: $\bar{X} \pm \boxed{Z_{\alpha/2}\frac{\sigma}{\sqrt{n}}}$ MOE 容许误差
- Sample size:

$$\text{MOE} = Z_{\alpha/2}\frac{\sigma}{\sqrt{n}} = 5$$ ←不是两倍

$$n = \left(Z_{\alpha/2} \cdot \sigma/\text{MOE}\right)^2 = (1.96 * 20/5)^2 = 61.5$$

Sample size = 62 取整

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1.3 Example 2 - Estimating a single proportion

- CI: $\left(\hat{p} - z_{\alpha/2}\sqrt{\frac{\hat{p}(1-\hat{p})}{n}}, \hat{p} + z_{\alpha/2}\sqrt{\frac{\hat{p}(1-\hat{p})}{n}}\right)$

$(\hat{p}\ \pm \text{MO}E)$ Point estimate ± Margin of Error

- Formula for sample size for estimation of a proportion is

$$n = \hat{p}(1-\hat{p})\left(Z_{\alpha/2}/\text{MOE}\right)^2$$

  - where n = the required sample size
  - $\hat{p}$ = the proportion estimation - here 0.30
  - *MOE* = margin of error here 0.05 上文中10%的一半

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注意点

1.3 Example 2： Estimating a single proportion

- Here we have: $n = 1.96^2 * 0.30 * (0.70)/0.05\hat{}2 = 322.7$
- "A sample of 323 patients with asthma will be required to obtain a 95% confidence interval of +/- 5% around a prevalence estimate of 30%. To allow for an expected 70% response rate to the questionnaire, a total of 480 questionnaires will be delivered."
- Note: The formula presented below is based on 'normal approximation methods', and, should not be applied when estimating percentages which are close to 0% or 100%. In these circumstances 'exact methods' should be used.

from hypothesis test

power

2.1 Power (1) – definition

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- A good test is one with low probability of committing a Type I error (i.e., α) and **high power (i.e., 1- β)**. power是啥
- The **power** of a hypothesis test is the **probability of making the correct decision if the alternative hypothesis is true**. 前提：Ha=T 含义：做出正确决定（即拒绝H0）的可能性
- The **power** of a hypothesis test is the probability of rejecting H0 when the alternative hypothesis Ha is true.

2.1 Power (1) – definition

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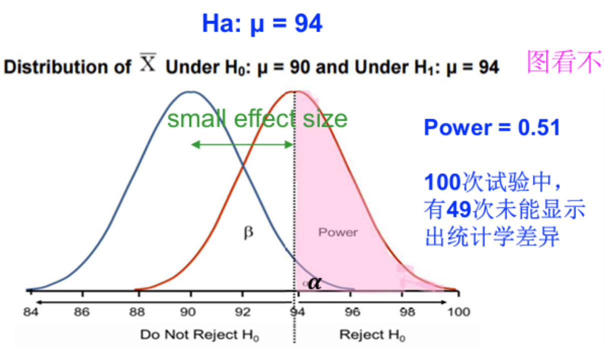
- Suppose we want to test the hypotheses at a α=0.05: H0: μ = 90 versus Ha: μ > 90. 单边 To test the hypotheses, suppose we select a sample of size n=100.
- 效应量 Effect size: We can think about our effect size as the importance of a certain effect. The larger the effect size, the more easily it can be seen by just looking.



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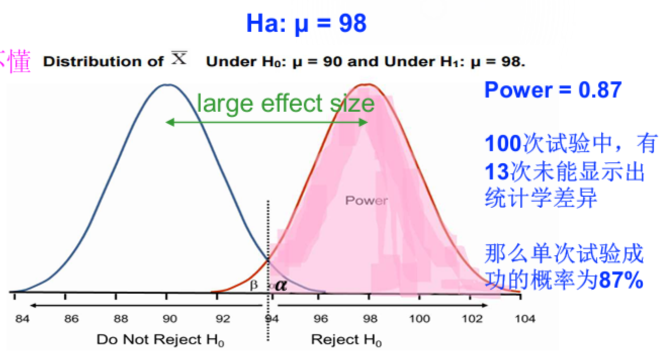

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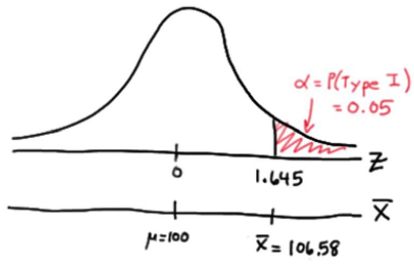
2.1 Power (2) – Example*

Let X denote the IQ of a randomly selected adult American. Assume, a bit unrealistically, that X is normally distributed with unknown mean μ and standard deviation 16.

Take a random sample of $n = 16$ students, after setting the probability of committing a Type I error at $\alpha = 0.05$, we can test the null hypothesis $H_0 : \mu = 100$ against the alternative hypothesis that $H_A : \mu > 100$.

What is the power of the hypothesis test if the true population mean were $\mu = 108$?

Answer: setting $\alpha = 0.05$, implies that we should reject the null hypothesis when the test statistic $Z \geq 1.645$, or equivalently, when the observed sample mean is 106.58 or greater:

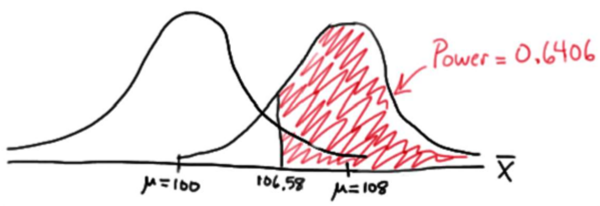


$$Z = \frac{\bar{X} - \mu}{\frac{\sigma}{\sqrt{n}}} \qquad \Rightarrow \bar{X} = \mu + Z\frac{\sigma}{\sqrt{n}} \qquad \bar{X} = 100 + 1.645\left(\frac{16}{\sqrt{16}}\right) = 106.58$$

2.1 Power (2) – Example*

$$\text{Power} = P(\bar{X} \geq 106.58 \text{ when } \mu = 108) = P\left(Z \geq \frac{106.58 - 108}{\frac{16}{\sqrt{16}}}\right) = P(Z \geq -0.36) = 1 - P(Z < -0.36)$$

We have determined that we have (only) a 64.06% chance of rejecting the null hypothesis H0:μ=100 in favor of the alternative hypothesis Ha:μ>100 if the true unknown population mean is in reality μ=108.



2.1 Power (3) – calculation*

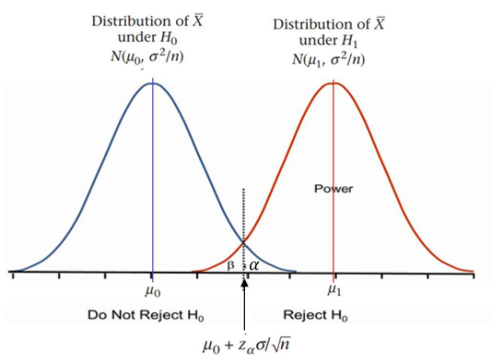
H0: $\mu = \mu_0$ vs. Ha: $\mu > \mu_0$

Power = P(reject H0|H0 false)

$= P(Z > z_{1-\alpha} | \mu = \mu_1)$

$= P\left(\frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}} > z_{1-\alpha} \middle| \mu = \mu_1\right)$

$= P(\bar{x} > \mu_0 + z_{1-\alpha}\sigma/\sqrt{n} | \mu = \mu_1)$



Under Ha, $\bar{X} \sim N(\mu_1, \sigma^2/n)$. Hence, after standardization,

Power = $1 - \Phi\left[\frac{\mu_0 + z_{1-\alpha}\frac{\sigma}{\sqrt{n}} - \mu_1}{\frac{\sigma}{\sqrt{n}}}\right] = 1 - \Phi[z_{1-\alpha} + \frac{\mu_0 - \mu_1}{\sigma}\sqrt{n}]$

power的比较

2.2 Calculating sample size

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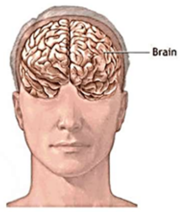
Let X denote the IQ of a randomly selected adult American. Assume, a bit unrealistically again, that X is normally distributed with unknown mean μ and (a strangely known) standard deviation of 16. This time, instead of taking a random sample of $n = 16$ students, let's increase the sample size to $n = 64$. And, while setting the probability of committing a Type I error to $\alpha = 0.05$, test the null hypothesis $H_0 : \mu = 100$ against the alternative hypothesis that $H_A : \mu > 100$.

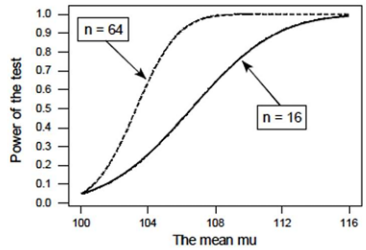


What is the power of the hypothesis test when $\mu = 108$, $\mu = 112$, and $\mu = 116$?

Answer: we can calculate the power of testing $H0: \mu = 100$ against $Ha: \mu > 100$ for 2 sample sizes (n=16 and n=64) and for 3 possible values of the mean (μ=108, μ=112, and μ=116). Here's a summary of our power calculations:

POWER	K(108)	K(112)	K(116)
$n = 16$	0.6406	0.9131	0.9909
$n = 64$	0.9907	0.9999...	0.999999...

pow ↑ ↑ ↑



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计算样本量

2.2 Calculating sample size

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- Recall Power = $1 - \Phi\left[\frac{\mu_0 + \frac{z_{1-\alpha}\sigma}{\sqrt{n}} - \mu_1}{\frac{\sigma}{\sqrt{n}}}\right] = 1 - \Phi[z_{1-\alpha} + \frac{\mu_0 - \mu_1}{\sigma}\sqrt{n}]$ 1-sided test
- The sample size computations depend on the level of significance α, the desired power of the test (equivalent to $1 - \beta$), the variability of the outcome, and the effect size.

? ? ? ? ? ? ? ? ?

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

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

if σ^2 is unknown, replace σ^2 by S^2

注意点

2.2 Calculating sample size

Q: "the larger sample size, the better" Is it true?

A: From confidence interval point of view, the larger sample size, the better.

But from hypothesis testing point of view, if sample size n is too large, then …

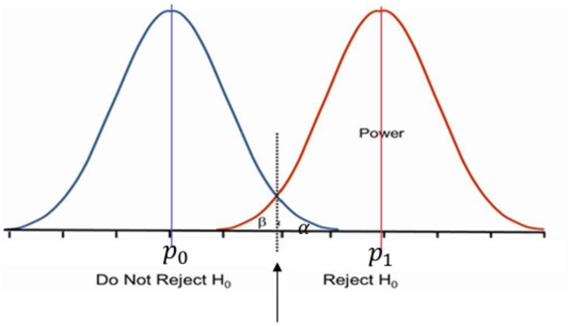
Sample size for one sample proportion one-sided-大样本

3.1 Sample size for one sample proportion – one-sided

(assuming large sample)

Under H0: $\hat{p} \sim N(p_0, \frac{p_0(1-p_0)}{n})$

Under Ha: $\hat{p} \sim N(p_1, \frac{p_1(1-p_1)}{n})$



$H_0: p = p_0 \ vs. \ H_a: p < or > p_0$

(we believe true $p = p_1$)

$$n = \frac{\left(Z_{1-\alpha}\sqrt{p_0(1-p_0)} + Z_{1-\beta}\sqrt{p_1(1-p_1)}\right)^2}{(p_1 - p_0)^2}$$

$$^*1-\beta = \Phi\left(\sqrt{\frac{p_0(1-p_0)}{p_1(1-p_1)}}\left(\frac{|p_1-p_0|\sqrt{n}}{\sqrt{p_0(1-p_0)}} - Z_{1-\alpha}\right)\right)$$

Φ是啥意思

Φ (x)是X~N (0， 1)时X的 分布函数 。 分布函数（英文Cumulative Distribution Function, 简称CDF）

Sample size for two-population mean-大样本

3.2 Sample size for two-population mean

$H_0: \mu_A - \mu_B = 0$
$H_1: \mu_A - \mu_B \neq 0$

The ratio between the sample sizes of the two groups is $\kappa = \frac{n_A}{n_B}$

看不懂

$$n_A = \kappa n_B \text{ and } n_B = \left(1 + \frac{1}{\kappa}\right)\left(\sigma \frac{z_{1-\alpha/2} + z_{1-\beta}}{\mu_A - \mu_B}\right)^2$$

$$1 - \beta = \Phi\left(z - z_{1-\alpha/2}\right) + \Phi\left(-z - z_{1-\alpha/2}\right) \quad , \quad z = \frac{\mu_A - \mu_B}{\sigma\sqrt{\frac{1}{n_A} + \frac{1}{n_B}}}$$

Sample size for two-population proportions-大样本

3.3 Sample size for two-population proportions

The ratio between the sample sizes of the two groups is $\kappa = \frac{n_A}{n_B}$

$$H_0: p_A - p_B = 0$$
$$H_1: p_A - p_B \neq 0$$

$$n_A = \kappa n_B \text{ and } n_B = \left(\frac{p_A(1-p_A)}{\kappa} + p_B(1-p_B)\right)\left(\frac{z_{1-\alpha/2} + z_{1-\beta}}{p_A - p_B}\right)^2$$

$$1 - \beta = \Phi\left(z - z_{1-\alpha/2}\right) + \Phi\left(-z - z_{1-\alpha/2}\right) \quad , \quad z = \frac{p_A - p_B}{\sqrt{\frac{p_A(1-p_A)}{n_A} + \frac{p_B(1-p_B)}{n_B}}}$$

Sample Size for Matched/Paired Samples

3.4 Sample Size for Matched/Paired Samples

- $H_0: d = 0 \ vs. \ H_a: d = d_1$ ？？？？？？？
- Sample size: same logic as one-sample case 作差

注意点

3.5 More about sample size calculation

- 任何试验，理论上设计方案需要先确定，样本量估计是在设计方案基础上。不同的设计方案，样本量的估计方法不同。
- 样本量估计与数据类型有关。
- 样本量估计需要与今后将要使用的统计方法的条件结合。今天学习的是特定分析下的样本量计算。
- 估算的样本量是最少需要的量，考虑可能出现的各种情况，在计划样本量时候应增加若干样本量例数（e.g., +20%）
- 多组设计时，一般要求各组的样本量相等。
- 研究有多效应指标时，样本量估计应对每个效应指标进行样本量估计，然后取最大值。或者只对主要指标进行样本量估计。

取最大值