Power and Sample Size
Bios 662

Michael G. Hudgens, Ph.D.

mhudgens@bios.unc.edu

http://www.bios.unc.edu/∼mhudgens

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Outline

- Introduction
- One sample:
  - continuous outcome, Z test
  - continuous outcome, t test
  - binary outcome, Z test
  - binary outcome, exact test

Introduction

- Read Chs 5.8 and 6.3.3. of text
- In a test of a hypothesis, we are testing whether some population parameter has a particular value

$$H_0 : \theta = \theta_0,$$

where θ_0 is a known constant

- Generally,

$$H_A : \theta \neq \theta_0$$

Introduction

- Once the data are collected, we will compute a statistic related to θ, say $S(\hat{\theta})$
- $S(\hat{\theta})$ is a random variable, since it is computed from a sample (and hence it has a probability distribution)

Power

$$\Pr[\text{Type I error}] = \alpha = \Pr[S(\hat{\theta}) \in C_\alpha | H_0]$$

$$\Pr[\text{Type II error}] = \beta = \Pr[S(\hat{\theta}) \notin C_\alpha | H_A]$$

$$Power = 1 - \beta = \Pr[S(\hat{\theta}) \in C_\alpha | H_A]$$

One sample Z test

- Example: One sample test
- Study: collect data on continuous outcome (Y) on N individuals
- $E(Y) = \mu$, $V(Y) = \sigma^2$

$$H_0 : \mu = \mu_0 \text{ vs } H_A : \mu > \mu_0$$

$$S(\hat{\theta}) = Z = \frac{\bar{Y} - \mu_0}{\sigma/\sqrt{N}}$$

One sample Z test

$$\Pr[S(\hat{\theta}) \in C_\alpha | H_0] = \Pr[Z > z_{1-\alpha} | H_0] = \alpha$$

$$\Pr[S(\hat{\theta}) \in C_\alpha | H_A] = \Pr[Z > z_{1-\alpha} | H_A] = 1 - \beta$$

- Choose a value $\mu_A \in H_A$
- Q: what sample size do we need to detect this alternative with $1 - \beta$ power?

One sample Z test

- Under $H_A : \mu = \mu_A$

$$Z' = \frac{\overline{Y} - \mu_A}{\sigma/\sqrt{N}} \sim N(0,1)$$

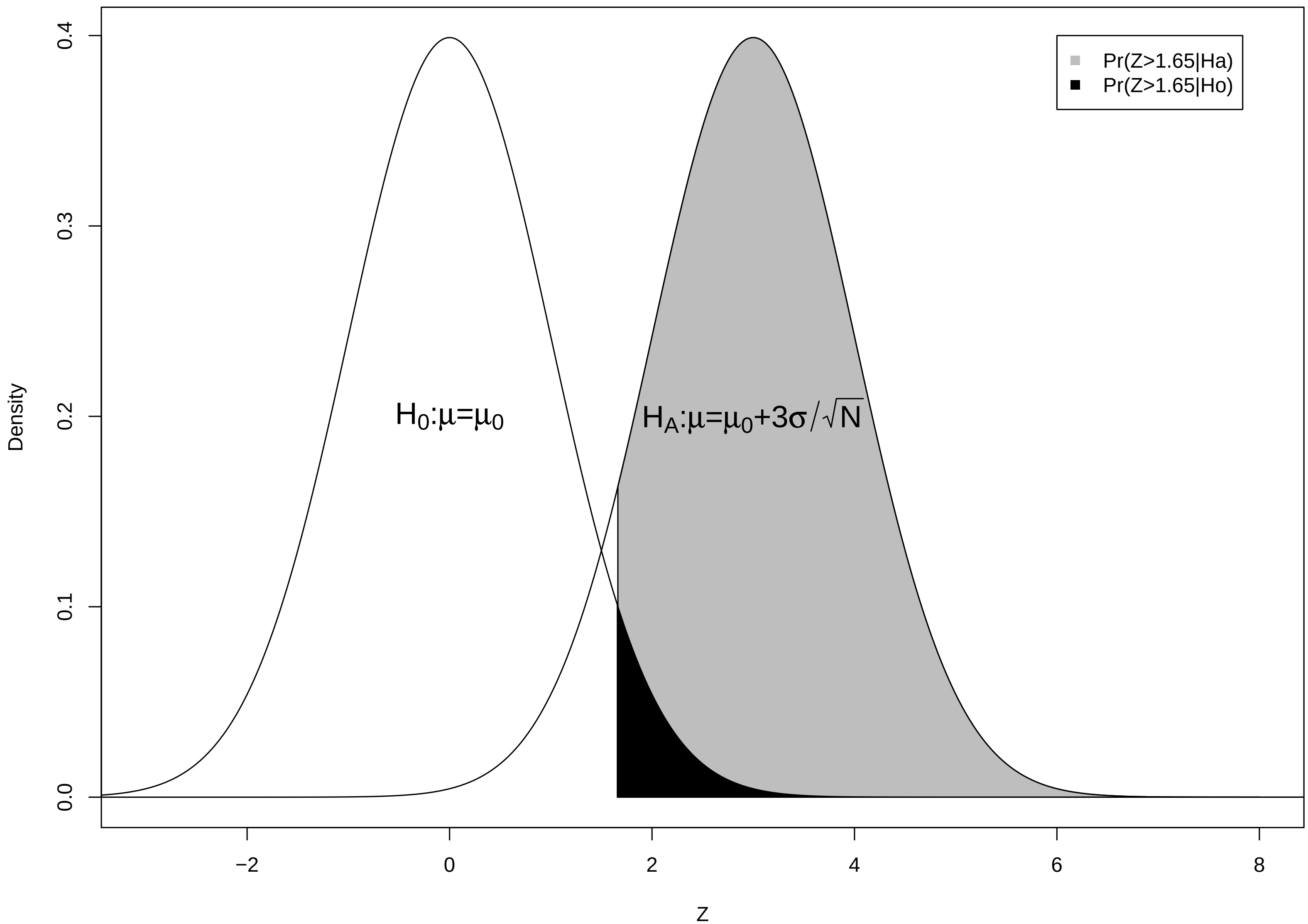
- Note

$$Z' + \frac{\mu_A - \mu_0}{\sigma/\sqrt{N}} = \frac{\overline{Y} - \mu_0}{\sigma/\sqrt{N}} = Z$$

- Thus

$$Z \sim N\left(\frac{\mu_A - \mu_0}{\sigma/\sqrt{N}}, 1\right)$$

Distribution of Z-statistic under H_0 and H_A



One sample Z test

- Power is given by

$$1-\beta = \Pr[Z > z_{1-\alpha} | \mu = \mu_A]$$

$$= \Pr[Z' > z_{1-\alpha} + \frac{\sqrt{N}(\mu_0 - \mu_A)}{\sigma} | \mu = \mu_A]$$

- Therefore

$$z_{1-\alpha} + \frac{\sqrt{N}(\mu_0 - \mu_A)}{\sigma} = z_\beta = -z_{1-\beta}$$

One sample Z test

- Equivalently

$$N = \frac{\sigma^2 (z_{1-\alpha} + z_{1-\beta})^2}{(\mu_0 - \mu_A)^2}$$

- For a two sided test

$$N = \frac{\sigma^2 (z_{1-\alpha/2} + z_{1-\beta})^2}{(\mu_0 - \mu_A)^2}$$

One sample Z test

- Equivalent form

$$N = \left(\frac{z_{1-\alpha/2} + z_{1-\beta}}{\Delta}\right)^2$$

where

$$\Delta = \frac{|\mu_0 - \mu_A|}{\sigma}$$

- Δ is called the *standardized distance* or *difference*

One sample Z test

- To apply this formula, we need to know α, β, $|\mu_0 - \mu_A|$, and σ^2
- Example: a study to determine the effect of a drug on blood pressure.
- BP measured, drug administered, and BP measured 2 hours later
- $Y = (BP_{after} - BP_{before})$

$$H_0 : \mu = 0 \text{ vs } H_A : \mu \neq 0$$

One sample Z test

- Choose $\alpha = 0.05$, $1 - \beta = 0.9$
- Estimate of σ^2: need data from literature or pilot study; note we need variance of difference after-before
- The choice of μ_A is a subject matter decision. In example: a drug that changes BP only 1 mmHg would not be of practical importance, but a drug that changes BP 5 mmHg might be of interest

One sample Z test

- Suppose $\alpha = 0.05$, $1 - \beta = .9$, $\sigma^2 = 225$, and $\mu_A = 5$

$$N = \frac{225(1.96 + 1.28)^2}{5^2} = 94.5 \approx 95$$

- Often compute N for various different values of α, β, σ^2, and μ_A
- Note $(1.96 + 1.28)^2 \approx 10.5$, so that

$$N \approx \frac{10.5}{\Delta^2}$$

One sample Z test

α	$1-\beta$	σ^2	μ_A	N	α	$1-\beta$	σ^2	μ_A	N
0.05	0.90	225	5	95	0.01	0.90	225	5	133
		225	6	66			225	6	93
		256	5	108			256	5	151
		256	6	75			256	6	105
0.05	0.80	225	5	71	0.01	0.80	225	5	105
		225	6	49			225	6	73
		256	5	81			256	5	119
		256	6	56			256	6	83

One sample Z test

$$\alpha \downarrow \Rightarrow N \uparrow$$

$$1 - \beta \uparrow \Rightarrow N \uparrow$$

$$\sigma^2 \uparrow \Rightarrow N \uparrow$$

$$|\mu_0 - \mu_A| \downarrow \Rightarrow N \uparrow$$

One sample Z test

- Sometimes N is fixed and we estimate the power of the test

$$N = \frac{\sigma^2(z_{1-\alpha/2} + z_{1-\beta})^2}{(\mu_0 - \mu_A)^2}$$

$$z_{1-\beta} = \frac{|\mu_0 - \mu_A|\sqrt{N}}{\sigma} - z_{1-\alpha/2}$$

$$1 - \beta = \Phi\left\{\frac{|\mu_0 - \mu_A|\sqrt{N}}{\sigma} - z_{1-\alpha/2}\right\}$$

One sample Z test

- Investigator says there are only 50 patients, $\alpha = .05$, $\sigma^2 = 225$, $|\mu_0 - \mu_A| = 5$

$$z_{1-\beta} = \frac{5\sqrt{50}}{15} - 1.96 = 0.40$$

$$1 - \beta = 0.6554$$

One sample t test

- In practice, σ is not know, so we use a t test instead of a Z test
- Results above should be viewed as approximations in this case
- Power of t test is as follows

One sample t test

- Need the following result: if $U \sim N(\lambda, 1)$ and $V \sim \chi^2_\nu$ where $U \perp V$, then

$$\frac{U}{\sqrt{V/\nu}} \sim t_{\nu,\lambda}$$

  i.e., a non-central t distribution with df ν and non-centrality parameter λ

One sample t test

- Consider $H_0 : \mu = 0$ versus $H_A : \mu = \mu_A$ for $\mu_A \neq 0$
- Under H_A,
$$\overline{Y} \sim N(\mu_A, \sigma^2/N)$$
- Thus
$$\frac{\overline{Y}\sqrt{N}}{\sigma} \sim N(\mu_A \frac{\sqrt{N}}{\sigma}, 1)$$
- Recall
$$\frac{(N-1)s^2}{\sigma^2} \sim \chi^2_{N-1}$$

One sample t test

- Since $\overline{Y} \perp s^2$,

$$T = \frac{\overline{Y}}{s/\sqrt{N}} \sim t_{N-1,\lambda}$$

where $\lambda = \mu_A\sqrt{N}/\sigma$

- So power of two-sided t-test for $\mu_A > 0$

$$\Pr[T \geq t_{N-1,0;1-\alpha/2}]$$

where $T \sim t_{N-1,\mu_A\sqrt{N}/\sigma}$

One sample t test: R

```
# by hand
> 1-pt(qt(.975,49), 49, 5/15*sqrt(50))
[1] 0.6370846

> power.t.test(n=50, sd=15, delta=5, type="one.sample")

     One-sample t test power calculation

              n = 50
          delta = 5
             sd = 15
      sig.level = 0.05
          power = 0.6370846
    alternative = two.sided
```

One sample t test: SAS

```
proc power;
      onesamplemeans
         mean   = 5
         ntotal = 50
         stddev = 15
         power  = .; run;
```

```
The POWER Procedure
One-sample t Test for Mean

     Fixed Scenario Elements
Distribution                Normal
Method                       Exact
Mean                             5
Standard Deviation              15
Total Sample Size               50
Number of Sides                  2
Null Mean                        0
Alpha                         0.05

Computed Power
Power
0.637
```

One sample Z test: Binary Outcome

- Null hypothesis

$$H_0 : \pi = \pi_0$$

- Test statistic

$$Z = \frac{\hat{p} - \pi_0}{\sqrt{\pi_0(1 - \pi_0)/N}}$$

- Sample size formula

$$N = \frac{\left(z_{1-\alpha/2}\sqrt{\pi_0(1 - \pi_0)} + z_{1-\beta}\sqrt{\pi_A(1 - \pi_A)}\right)^2}{(\pi_A - \pi_0)^2}$$

One sample Z test: Binary Outcome

- Example: Study of risk of breast cancer in sibs.
- Prevalence in general population of women 50-54 years old: 2%
- Sample sisters of women with breast cancer

$$H_0 : \pi = 0.02 \text{ vs } H_A : \pi \neq .02$$

One sample Z test: Binary Outcome

- Suppose $\alpha = .05$, $1 - \beta = .9$, $\pi_A = .05$
- Then

$$N = \frac{\left(1.96\sqrt{.02(.98)} + 1.28\sqrt{.05(.95)}\right)^2}{(.05 - .02)^2} = 340.65 \approx 341$$

One sample Z test with binary outcome: SAS

```
proc power;
 onesamplefreq test = z
  method = normal
  nullp = .02
  p = .05
  power = .9
  ntotal = .;
run;
```

```
The POWER Procedure
Z Test for Binomial Proportion

          Fixed Scenario Elements
Method                     Normal approximation
Null Proportion                            0.02
Binomial Proportion                        0.05
Nominal Power                               0.9
Number of Sides                               2
Alpha                                      0.05

Actual        N
 Power    Total
 0.900      341
```

One sample exact test: binary outcome

- What is power of exact test?
- Let Y, the number of successes, be test statistic.
- Under H_0, $Y \sim Binomial(N, \pi_0)$

  Under H_A, $Y \sim Binomial(N, \pi_A)$
- Power

$$\Pr[Y \geq y_{1-\alpha/2} | \pi = \pi_A] + \Pr[Y \leq y_{\alpha/2} | \pi = \pi_A]$$

where $y_{\alpha/2}$ and $y_{1-\alpha/2}$ determined as in Counting Data slides

One sample exact test: binary outcome

- Exact example. Suppose $N = 20$, $\pi_0 = .2$, $\pi_A = .5$, $\alpha = 0.05$. What is exact power of two-sided test?
- Based on CDF of $Binomial(20, .2)$, choose $y_{\alpha/2} = 0$ and $y_{1-\alpha/2} = 9$ such that power equals

$$\Pr[Y \geq 9|\pi = .5] + \Pr[Y \leq 0|\pi = .5] = 0.748$$

One sample exact test, binary outcome: SAS

```
proc power;
 onesamplefreq test = exact
  method = exact
  nullp = .2
  p = .5
  power = .
  ntotal = 20;
run;
```

```
          Computed Power

 Lower    Upper
 Crit     Crit     Actual
  Val      Val      Alpha    Power

    0        9     0.0215    0.748
```