Universidad del Cauca

ERASMUS UNIVERSITEIT ROTTERDAM

ANALYSIS OF CATEGORICAL OUTCOMES

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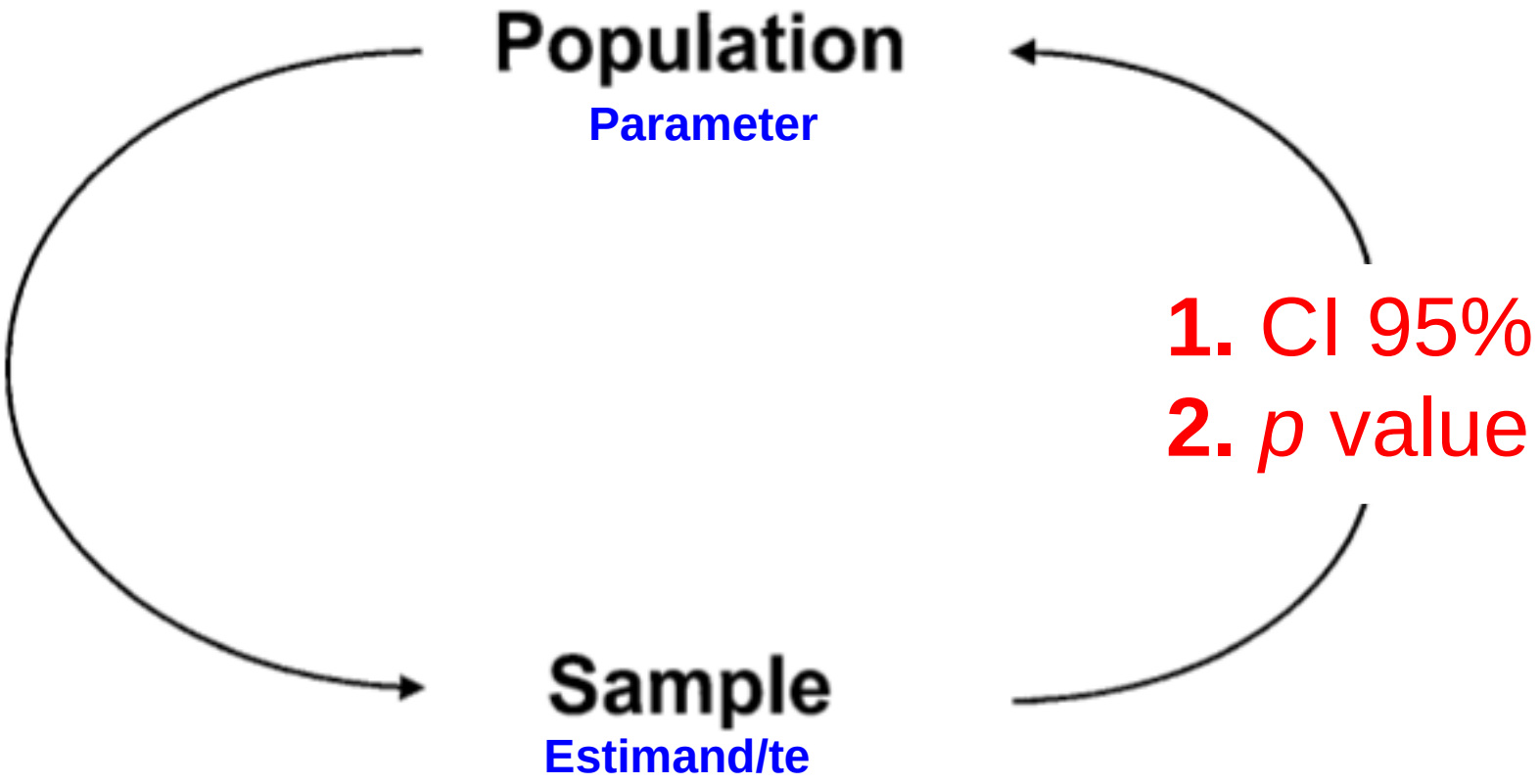

Population
Parameter
Sample
Estimand/te
1. CI 95%
2. p value

CI95% for a proportion

- A binary variable X of two categories: 1 / 0
- For the sample the proportion is p
- Useful if n is large

$$\sigma_{(p)} = \sqrt{\pi(1-\pi)}$$

$$DE_{(p)} = \sqrt{p(1-p)} \qquad se_{(p)} = \sqrt{\frac{p(1-p)}{n}}$$

$$CI95\% = p \pm 1.96\sqrt{\frac{p(1-p)}{n}}$$

Example,

- To estimate the prevalence of obesity in faculty students
  - n = 200
  - p = 30% = 0,3

$$DE(p) = \sqrt{p(1-p)} = \sqrt{0{,}3(1-0{,}3)} = 0{,}458$$

$$SE(p) = \sqrt{\frac{p(1-p)}{n}} = \sqrt{\frac{0{,}3(0{,}7)}{200}} = 0{,}032$$

$$CI95\% = p \pm 1.96\left(SE(p)\right) = 0{,}3 \pm 1{,}96(0{,}032)$$

$$CI95\% = 0{,}3\ [0{,}237 - 0{,}362] = [23{,}7\% - 36{,}2\%]$$

Comparing proportions

- Pediatric AIDS Clinical Trial Group (ACTG)
  - “We conducted a randomized, double-blinded, placebo- controlled trial of the efficacy and safety of zidovudine (AZT) in reducing the risk of maternal-infant HIV transmission”
  - 363 pregnant women with HIV were randomized to AZT or placebo (18 postnatal months)

    - 180 AZT HIV+ 13 children
    - 183 Placebo HIV+ 40 children

Conner, E., et al. (1994). Reduction of maternal-infant transmission of human immunodeficiency virus type 1 with zidovudine treatment, *New England Journal of Medicine* 331: 18.

Risk of transmission (CI 18 months)

	HIV +	HIV -	Total
AZT	13	167	**180**
Placebo	40	143	**183**
Total	**53**	**310**	**363**

CI_{AZT} = 13/180 = 7%

CI_{PLAC} = 40/183 = 22%

Cumulative incidence and CI 95%

- CI_{AZT} = 13/180 = 7% CI95% [3.9 - 12]
- CI_{PLAC} = 40/183 = 22% CI95% [16 - 28]

- 95% confidence intervals are calculated following the binomial distribution (if n is large it approaches normal)

The question is: Is this difference significant or can it be explained by chance (random error)?

Chi^2 test

	HIV +	HIV -	Total
AZT	13	167	**180**
Placebo	40	143	**183**
Total	**53**	**310**	**363**

What would they be then?
the "expected" values
if the H0 were true?

- Test that can be extended to two or more proportions
- It is based on,
  - "Expected" values and "observed" values
  - Expected: If H0 were true
  - Is there a relationship between columns and rows?

$$\chi^2 = \sum_{4\ cells} \frac{(0 - E)^2}{E}$$

"Observed" vs "expected" if H0 were true

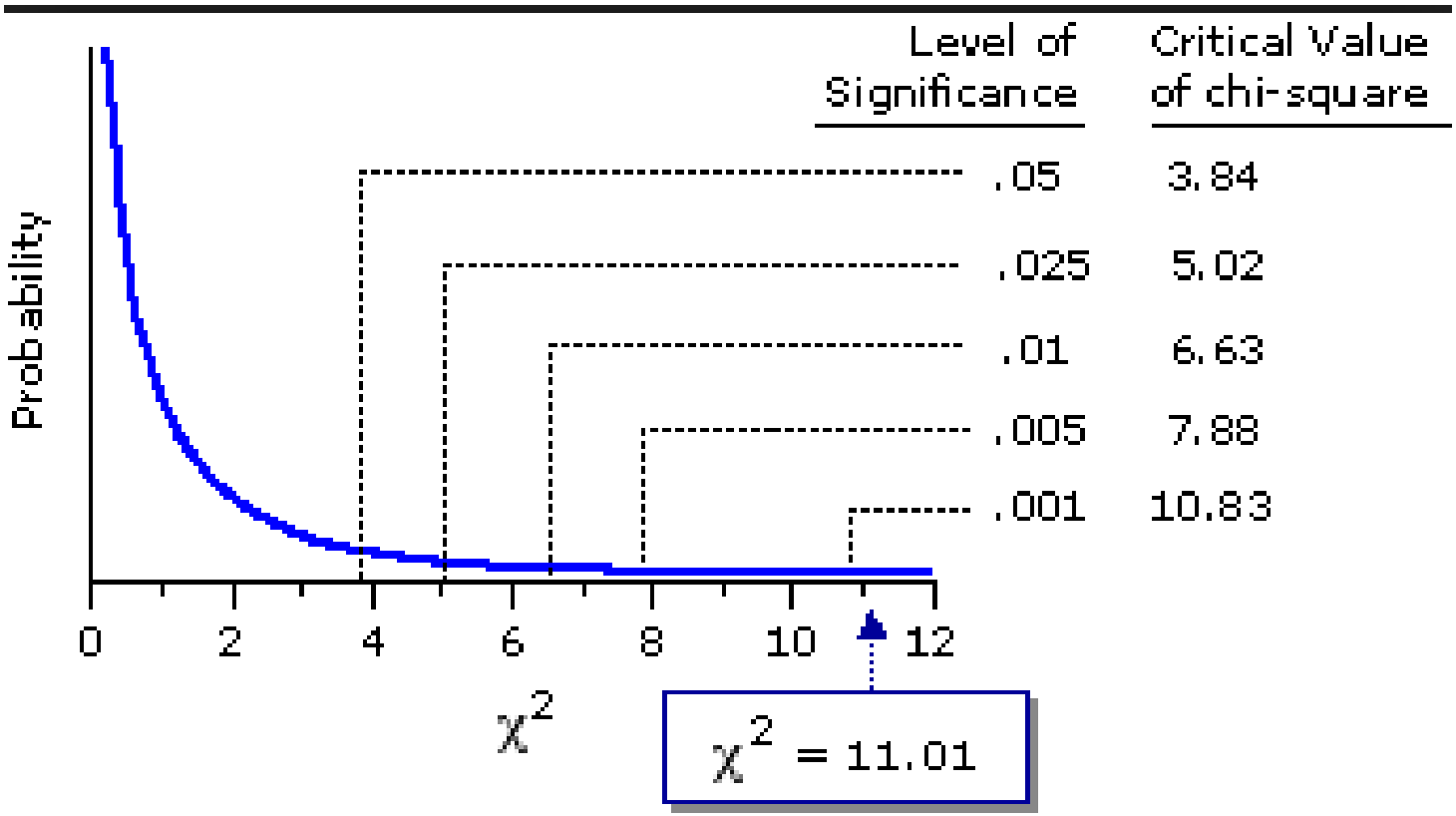
	HIV +	HIV -	Total
AZT	13	167	**180**
Placebo	40	143	**183**
Total	**53**	**310**	**363**

$$\chi^2 = \sum_{4\ cells} \frac{(0 - E)^2}{E}$$

"expected"
if the H0 were true

	HIV +	HIV -	Total
AZT	**26**	**154**	180
Placebo	**27**	**156**	183
Total	53	310	363

P value for Chi^2 test



- CI_{AZT} = 13/180 = 7% CI95% [3.9 - 12]
- CI_{PLAC} = 40/183 = 22% CI95% [16 - 28]

- P value for Chi^2 test = 0.0001

- There is sufficient evidence to reject H0 (equality)
- I accept H1 : There are “statistically significant” differences between AZT and Placebo

Example 2,

- Food poisoning occurred at a certain site. Some believe that the origin was chicken sandwiches

		Food poisoning		
Exposure		**Yes**	**No**	**Total**
	Sandwich	a	b	**a+b**
	Other food	c	d	**c+d**
	Total	**a+c**	**b+d**	**a+b+c+d**

$H_0: \quad P_{sand} = P_{otro}$

$H_1: \quad P_{sand} \neq P_{otro}$

Food poisoning

Exposure		Si	No	Total
	Sandwich	109	116	**225**
	Otro alimento	4	34	**38**
	Total	**113**	**150**	**263**

$p_{sand} = 109/225 = 48\%$

$p_{otro} = 4/38 = 10\%$

P value for Chi^2 test = 0.001

Fisher's exact test

- The chi2 test is based on a normal approximation
- Works well if the samples are large

- Solution,
  - Fisher's exact test
  - Difficult manual calculation
  - It has no approximations and delivers an "exact" p-value
  - Useful in small samples

Fisher's exact test

- 65 pregnant patients – classified as high risk for hypertension in pregnancy – are randomized in the third trimester to Aspirin 100 mg/day versus placebo to assess the effect on the occurrence of hypertension

	HT +	Non HT	Total
ASA 100	4	30	**34**
Placebo	11	20	**31**
Total	**15**	**50**	**65**

CI_{ASA} = 4/34 = 12%

CI_{PLAC} = 11/31 = 35%

Schiff, E. et al. The use of aspirin to prevent pregnancy-induced hypertension and lower the ratio of thromboxane A2 to prostacyclin in relatively high risk pregnancies. *New England Journal of Medicine,* 321, 6.

Fisher's exact test

	HT +	Non HT	Total
ASA 100	4	30	**34**
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Total	**15**	**50**	**65**

CI_{ASA} = 4/34 = 12%

CI_{PLAC} = 11/31 = 35%

- Fisher *p value* = 0.0378
- Chi^2 *p value* = 0.0234

Both tests reject H0

Test for variables with more than two categories

	Stratum 1	Stratum 2	Stratum 3	Total
Male	51	95	38	**184**
Female	17	101	75	**193**
Total	**68**	**196**	**113**	**377**

- The Chi2 test can be extended to variables with more than two categories
  - H0: Variables are independent
  - H1: Variables are related (or depend in some category on one another)

Test for variables with more than two categories

	Stratum 1	Stratum 2	Stratum 3	Total
Male	51	95	38	**184**
Female	17	101	75	**193**
Total	**68**	**196**	**113**	**377**

- Chi^2 p value = 0.000

- I have enough evidence to reject the H0 of independence
- They are significantly related