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Section E

The Sample Proportion as a Summary Measure for Binary Outcomes and the CLT

Proportions (p)

- Proportion of individuals with health insurance
- Proportion of patients who became infected
- Proportion of patients who are cured
- Proportion of individuals who are hypertensive
- Proportion of individuals positive on a blood test
- Proportion of adverse drug reactions
- Proportion of premature infants who survive

Proportions (p)

- For each individual in the study, we record a binary outcome (Yes/No; Success/Failure) rather than a continuous measurement

Proportions (p)

- Compute a sample proportion, $\hat{p}$ (pronounced "p-hat"), by taking observed number of "yes" responses divided by total sample size
  - This is the key summary measure for binary data, analogous to a mean for continuous data
  - There is a formula for the standard deviation of a proportion, but the quantity lacks the "physical interpretability" that it has for continuous data

Example 1

- Proportion of dialysis patients with national insurance in 12 countries (only six shown..)[1]

EXHIBIT 1
Descriptive Measures Of The Prevalent Cross-Sectional Patient Sample, Dialysis Patients In Twelve Countries, 2002–2004

	A/NZ (n = 561)	BEL (n = 468)	CAN (n = 503	FRA (n = 481)	GER (n = 524)	ITA (n = 540)
Mean age (years)	59.9 (14.7)	66.2 (13.4)	62.1 (14.7)	64.1 (14.5)	61.7 (14.1)	64 (13.7)
Minority[a]	21.5%	5.3%	18.7%	7.1%	0.4%	0.4%
Income ($US)						
<$20,000	85.0%	73.4%	71.8%	67.0%	59.7%	78.3%
$20,000–$39,000	9.1	17.5	20.8	21.8	27.1	17.4
≥$40,000	5.9	9.1	7.4	11.2	13.1	4.2
Insurance type						
National only	69.8%	74.1%	79.6%	45.5%	95.4%	99.6%
Private only	5.4	0.4	0.2	0.2	2.9	0.0
Mean number of comorbid conditions[b]	3.7 (2)	3.9 (2.1)	4.1 (2.1)	3.1 (1.9)	3.4 (2.1)	2.7 (1.9)
Mean number of prescribed medications	8.7 (3.6)	9.9 (4.1)	12.6 (4.8)	7.7 (3.5)	9.7 (3.5)	6.4 (3.6)

- Example: Canada: $\hat{p} = \frac{400}{503} = 0.796$

Notes: [1] Hirth, R., et al. (2008). Out-of-pocket spending and medication adherence among dialysis patients in twelve countries, *Health Affairs*, 27 (1).

Example 2

- Maternal/infant transmission of HIV[1]

- HIV-infection status was known for 363 births (180 in the zidovudine [AZT] group and 183 in the placebo group); thirteen infants in the zidovudine group and 40 in the placebo group were HIV-infected

$$\hat{p}_{AZT} = \frac{13}{180} = 0.07 = 7\%$$

$$\hat{p}_{PLAC} = \frac{40}{183} = 0.22 = 22\%$$

Notes: [1]Spector, S., et al. (1994). A controlled trial of intravenous immune globulin for the prevention of serious bacterial infections in children receiving zidovudine for advanced human immunodeficiency virus infection, *New England Journal of Medicine* 331 (18).

Proportions (p)

- What is the sampling behavior of a sample proportion?

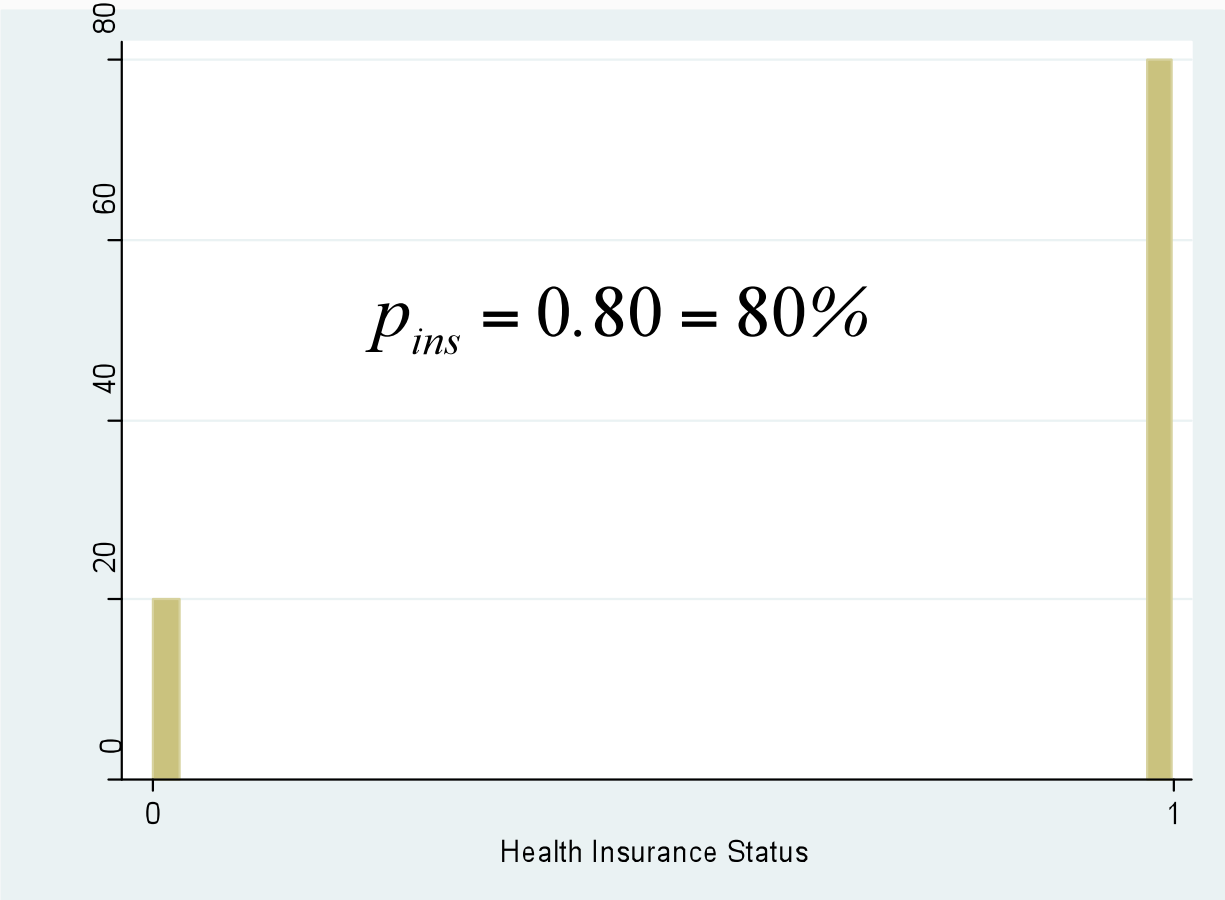
- In other words, how do sample proportions, estimated from random samples of the same size from the same population, behave?

Proportions (p)

- Suppose we have a population in which 80% of persons have some form of health insurance and 20% have no health insurance

Example: Health Insurance Coverage

- Assume the population distribution is given by the following:

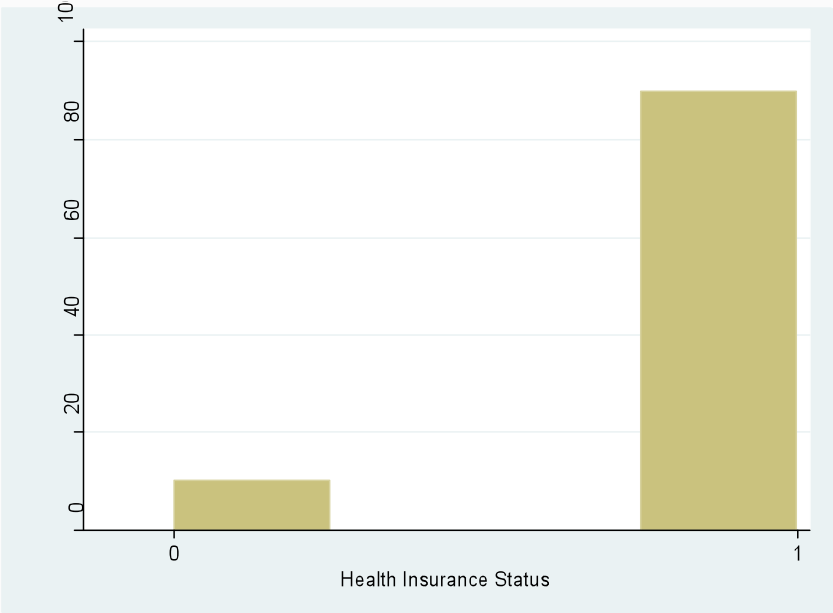


Example: Health Insurance Coverage

- Suppose we had all the time in the world (leftover from last time)
- We decide to do another set of experiments
- We are going to take 500 separate random samples from this population, each with 20 subjects
- For each of the 500 samples, we will plot a histogram of the sample proportion of insured individuals and record the sample proportion
- Ready, set, go . . .

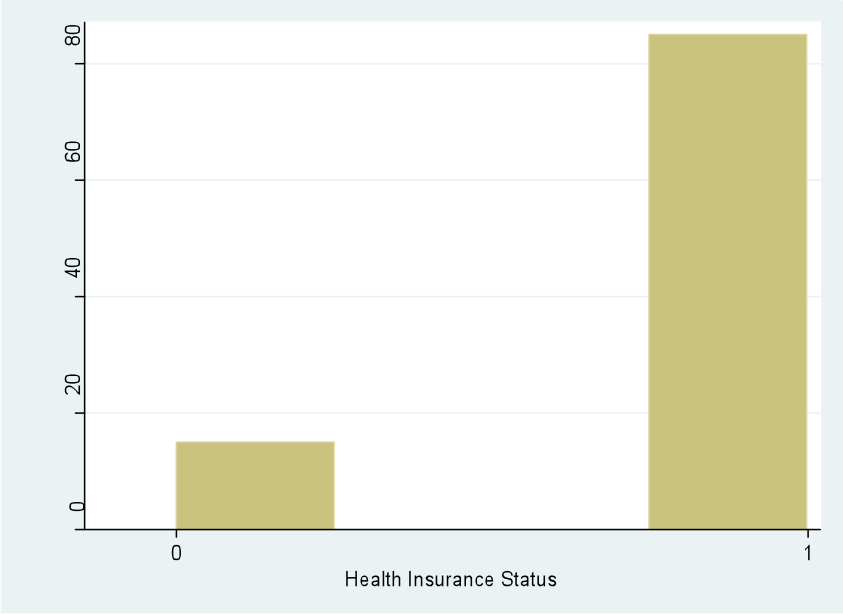
Random Samples

- Sample 1: $n = 20$



$$\hat{p}_{ins} = 0.90 = 90\%$$

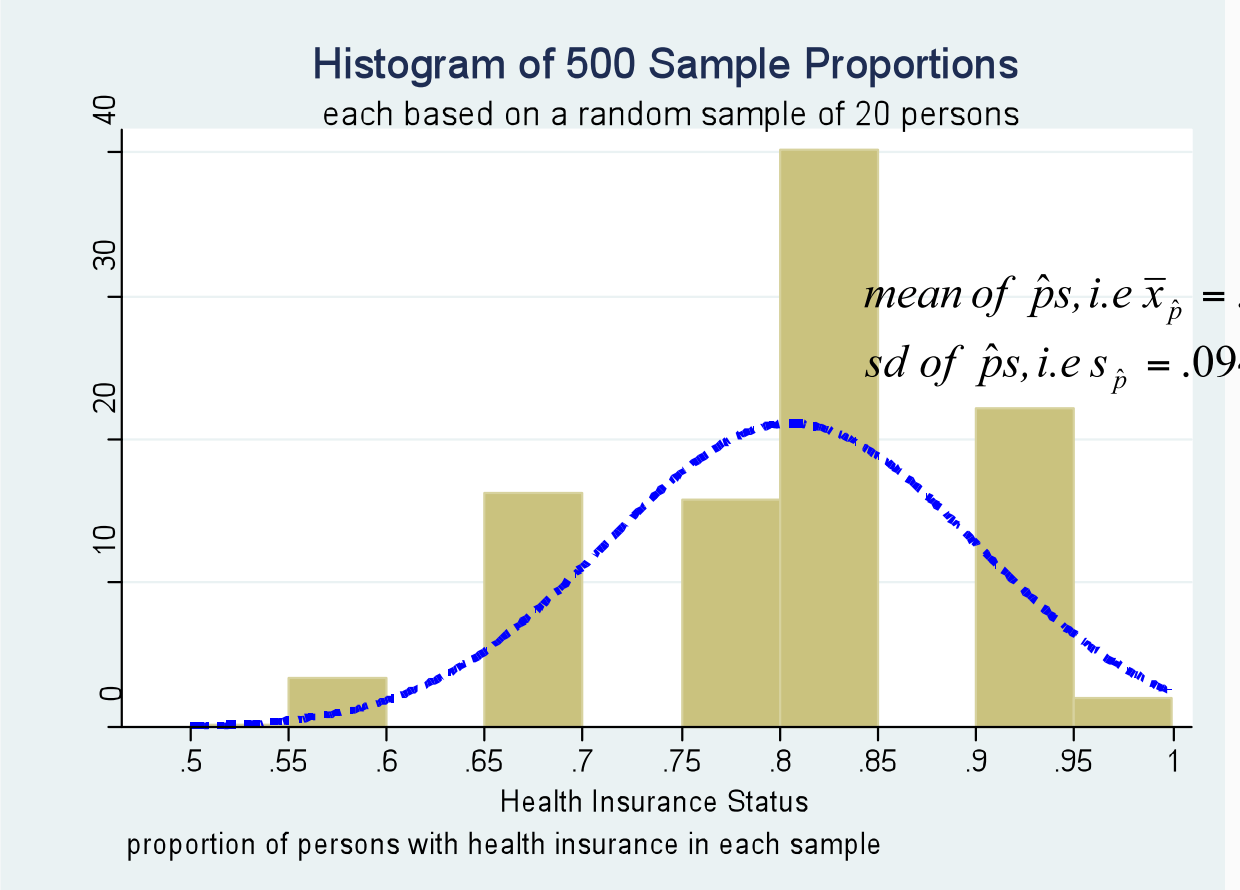
- Sample 2: $n = 20$



$$\hat{p}_{ins} = 0.85 = 85\%$$

Estimated Sampling Distribution

- So we did this 500 times: now let's look at a histogram of the 500 proportions

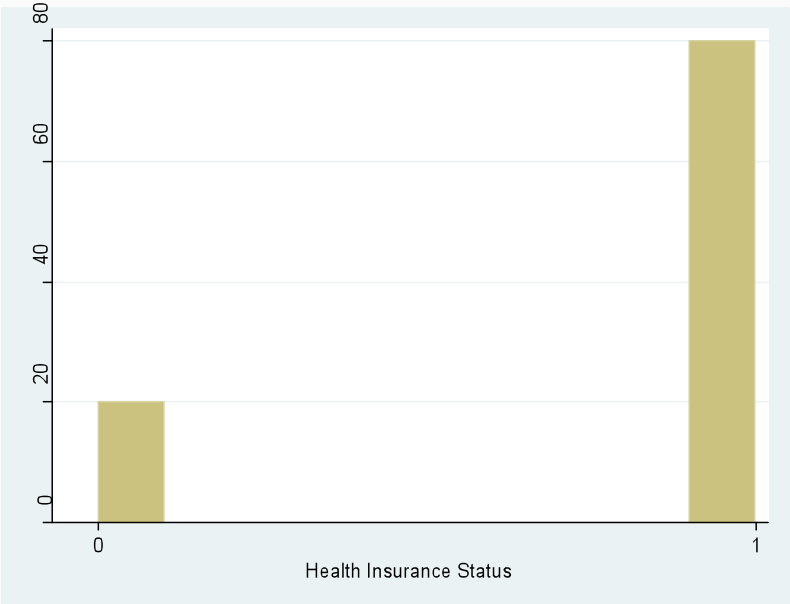


Example: Health Insurance Coverage

- We decide to do one more experiment
- We are going to take 500 separate random samples from this population, each with 100 subjects
- For each of the 500 samples, we will plot a histogram of the sample proportioned of insured individuals and record the sample proportion
- Ready, set, go . . .

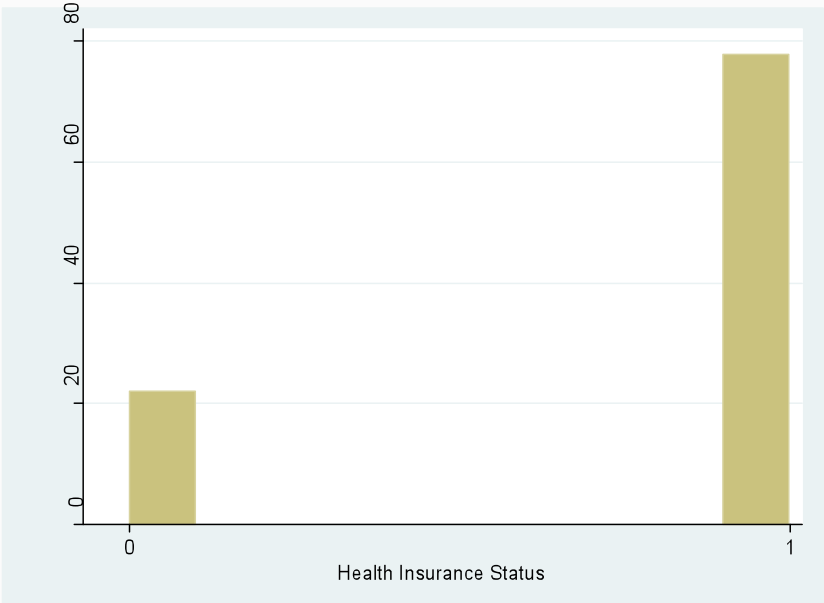
Random Samples

- Sample 1: n = 100



$$\hat{p}_{ins} = 0.80 = 80\%$$

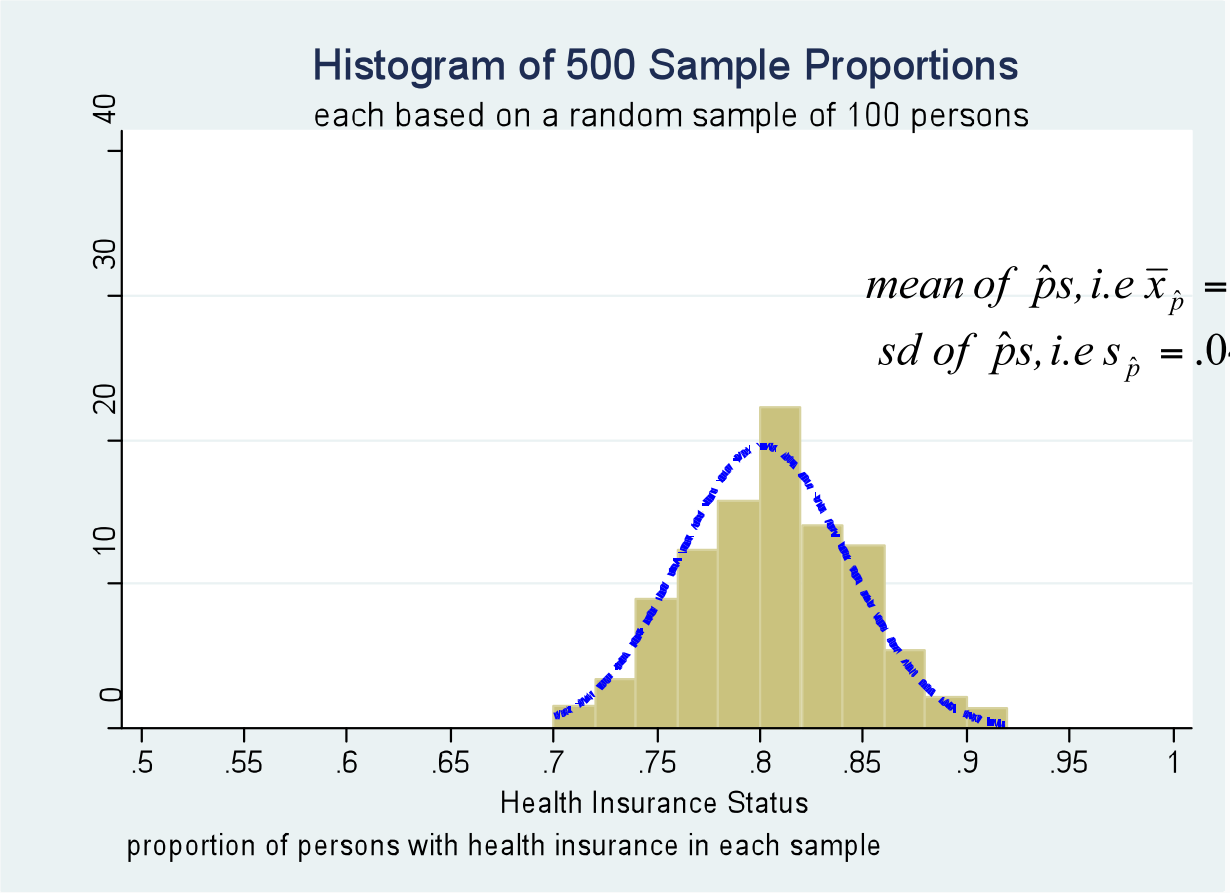
- Sample 2: n = 100



$$\hat{p}_{ins} = 0.78 = 78\%$$

Example: Blood Pressure of Males

- So we did this 500 times: now let's look at a histogram of the 500 proportions

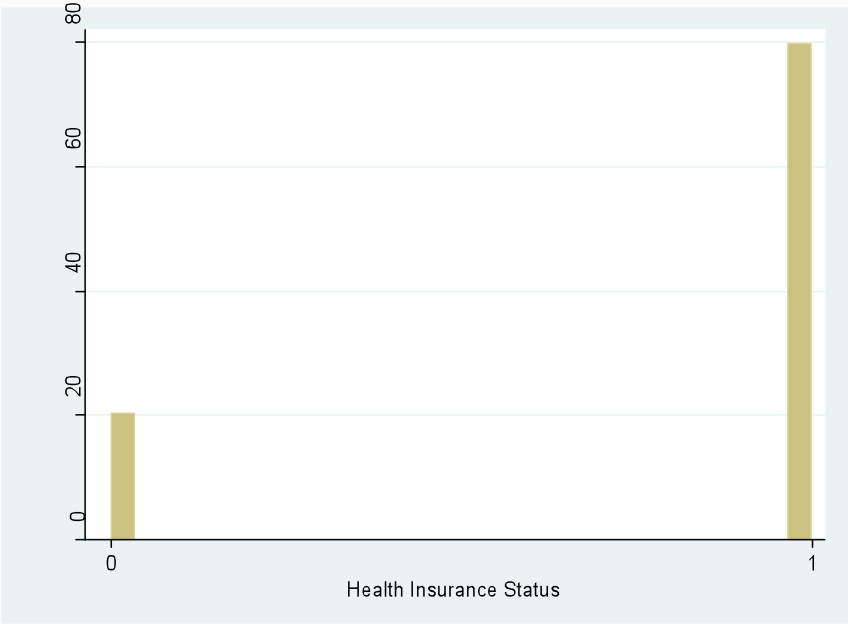


Example: Health Insurance Coverage

- We decide to do one more experiment
- We are going to take 500 separate random samples from this population, each with 1,000 subjects
- For each of the 500 samples, we will plot a histogram of the sample proportioned of insured individuals, and record the sample proportion
- Ready, set, go . . .

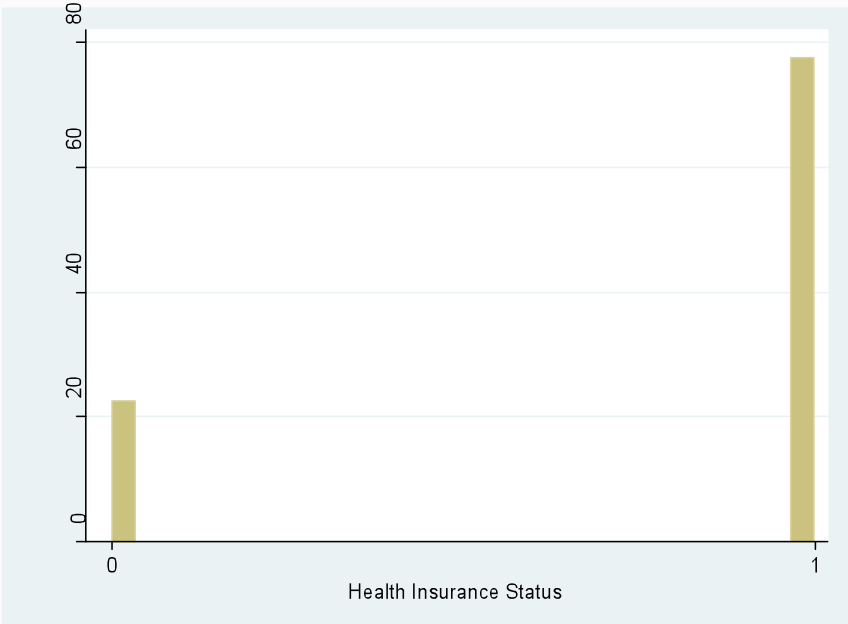
Random Samples

- Sample 1: n = 1,000



$$\hat{p}_{ins} = 0.798 = 79.8\%$$

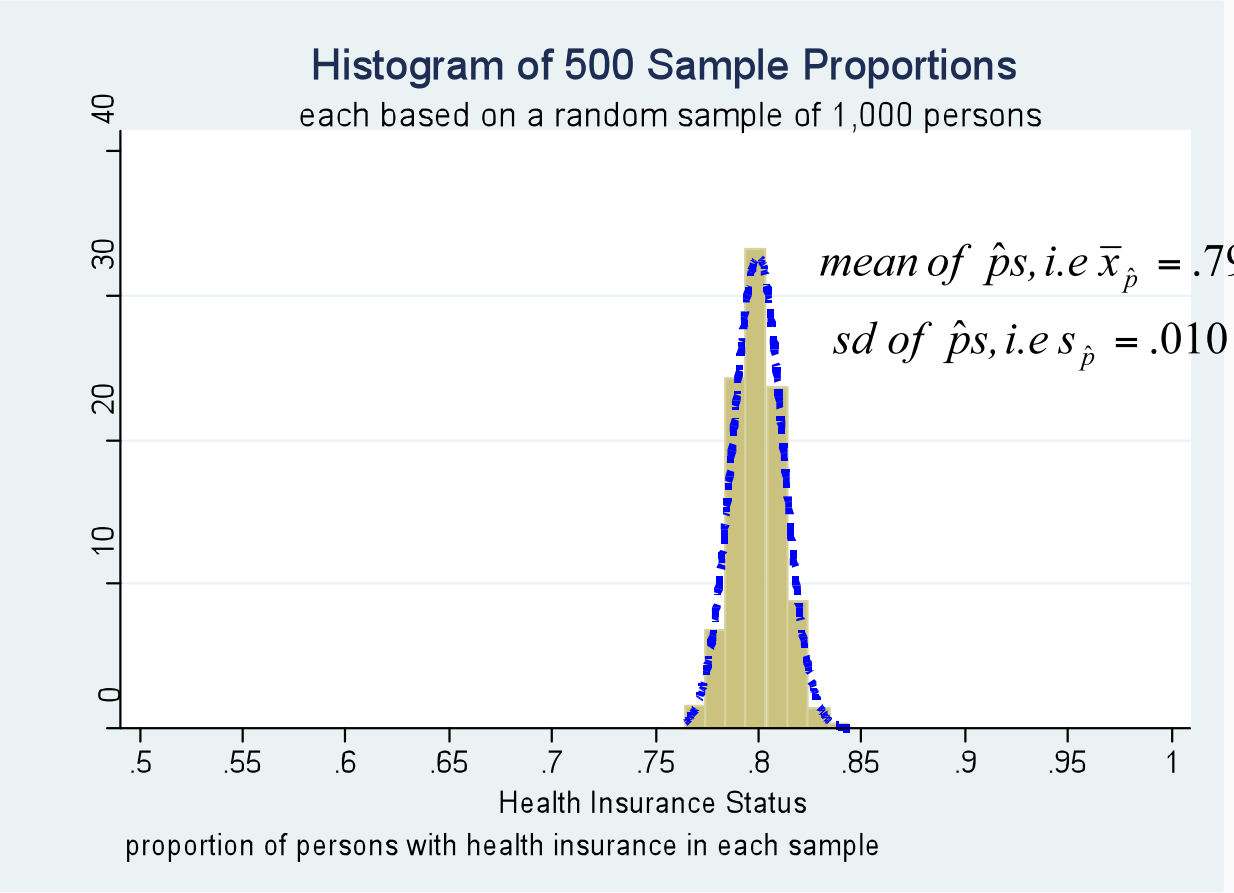
- Sample 2: n = 100



$$\hat{p}_{ins} = 0.777 = 77.7\%$$

Example: Blood Pressure of Males

- So we did this 500 times: now let's look at a histogram of the 500 proportions



Example 2: Hospital Length of Stay

- Let's review the results
- True proportion of insured: p = 0.80
- Results from 500 random samples:

Sample Sizes	Means of 500 Sample Proportions	SD of 500 Sample Proportions	Shape of Distribution of 500 Sample Proportions
n = 20	0.805	0.094	Approaching normal?
n = 100	0.801	0.041	Approximately normal
n = 1,000	0.799	0.012	Approximately normal

Example 2: Hospital Length of Stay

- Let's review the results