

Motivation

- Why learn about Experimental Design?
- Do I need to collaborate with a statistician, or I can analyze my data using available software?
- Hmmmm...



or



?

Clinical Trial Example

- A clinical trial is designed to estimate the efficacy of an experimental drug (D) compared to placebo (P) in congestive heart failure (CHF)
- Efficacy measure: the rate of change per week in distance walked after being administered therapy (D or P)
 - Baseline measure of left ventricular ejection fraction (LVEF) --- the lower the LVEF, the more serious CHF
 - 2 investigators
- Design and Analysis Tandem

Business Example

- A manufacturer is interested in **maximizing** the tensile strength of a new synthetic fiber that will be used to make cloth for men's shirt.
- From previous tests, the manufacturer knows:
 - the strength is affected by the **percentage of cotton** in the fiber
 - The **range** of the percentage is 10% to 40%

Statistical Inference

Statistical Inference

- Methods for drawing conclusions about a population from sample data are called statistical inference
- Methods
 1. Confidence Intervals - estimating a value of a population parameter
 2. Tests of significance - assess evidence for a claim about a population
- Inference is appropriate when data are produced by either
 - a random sample or
 - a randomized experiment

Estimation

The sample mean \bar{x}

- Central Limit Theorem: \bar{x} has a distribution that is approximately normal
- The mean of the sampling distribution is the same as the mean of the population μ
- The standard deviation of the sampling distribution is
$$\sigma / \sqrt{n}$$
- \bar{x} is an unbiased estimator of μ

The z statistic

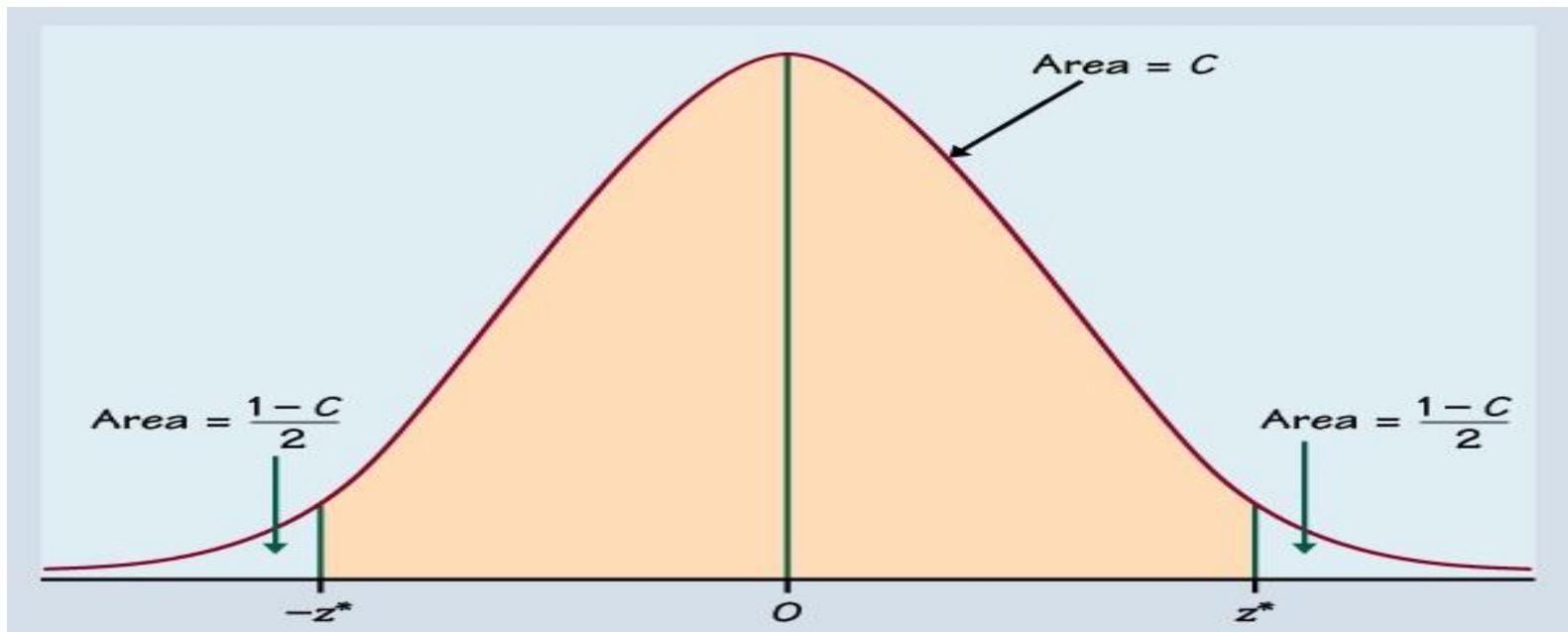
Standardize \bar{x}

$$z = \frac{\bar{x} - \mu}{\sigma / \sqrt{n}}$$

z has the standard normal distribution $N(0,1)$

The level C confidence interval for μ is $\bar{x} \pm z^* \frac{\sigma}{\sqrt{n}}$

Find z^* so that the central area on the normal curve has probability C



Margin of error

A level C confidence interval has the form:

$$\text{estimate} \pm \text{margin of error}$$

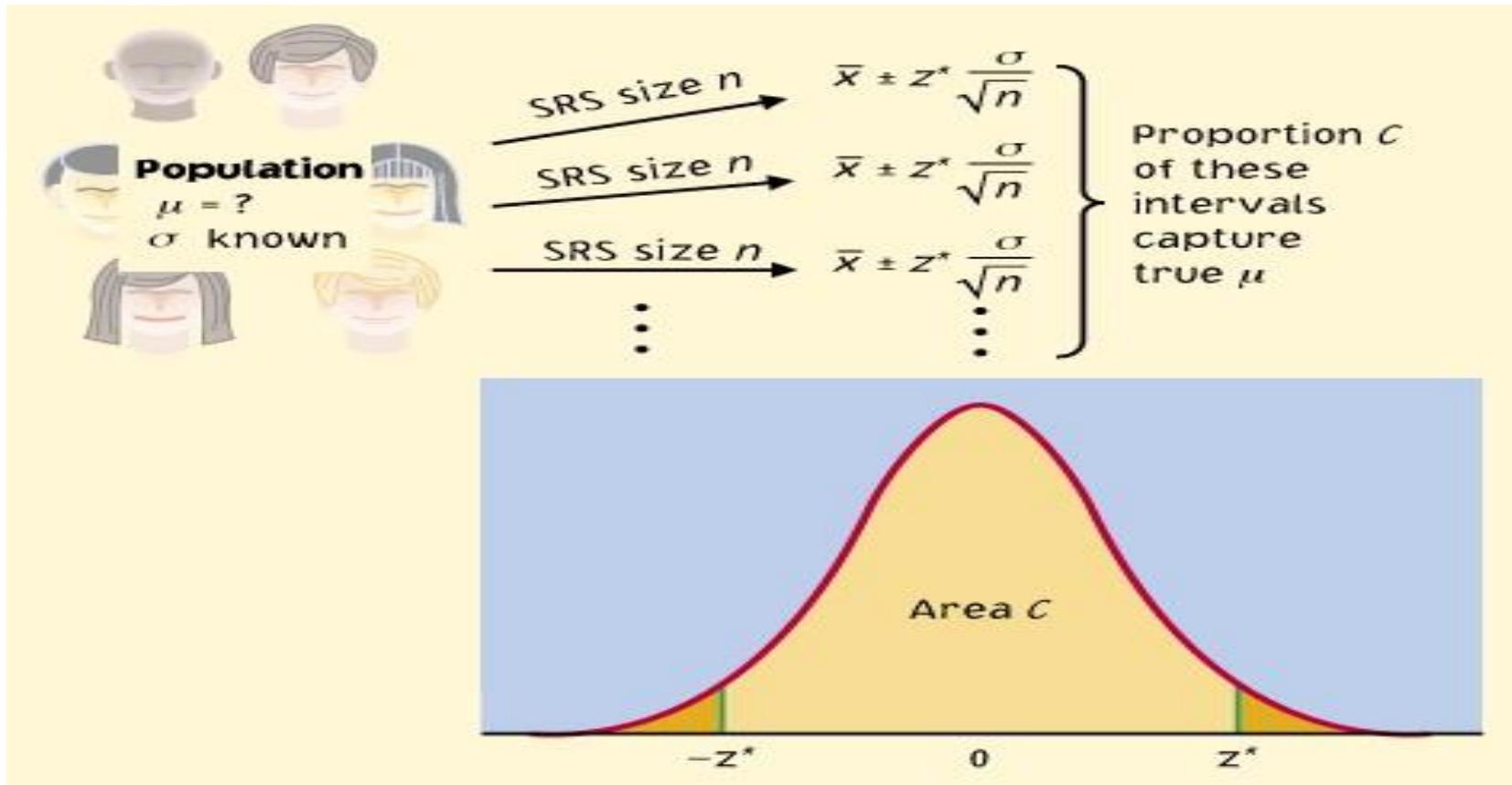
- The estimate is \bar{x}
- The margin of error shows how accurate we believe our estimate is
- The confidence level C , shows how confident we are that the procedure will catch the true population mean μ

Example:

The margin of error is usually reported with poll results

<http://www.pollingreport.com/>

The idea



Sample size

- You can have *both* high confidence and a small margin of error if the sample size is adequate
- Choose the sample size in advance
- The confidence interval for a population mean will have a specified margin of error m when the sample size is

$$n = \left(\frac{z^* \sigma}{m} \right)^2$$

Example

A test for the level of potassium in the blood is not perfectly precise. Moreover, the actual potassium level varies from day to day. Suppose that repeated measurements for the same person on different days vary normally with $\sigma = 0.2$.

a) If three measurements were taken on different days and the mean result is $\bar{x} = 3.2$ what is the 90% C.I. for the mean potassium level?

$$\bar{x} \pm z * \frac{\sigma}{\sqrt{n}} = 3.2 \pm 1.64 * 0.2 / \sqrt{3} = (3.01, 3.38)$$

b) How large a sample of would be needed to estimate μ within ± 0.05 with 90% confidence?

$$n = \left(\frac{z * \sigma}{m} \right)^2 = \left(\frac{1.64 * 0.2}{0.05} \right)^2 = 43$$

Cautions

- The data must be an random sample from the population
- Formula is not correct for more complex designs (such as stratified or cluster)
- The population must be normal or the sample size must be large
- You must know σ
- Outliers can have a large effect on the confidence interval

Example

- Determine the sample size necessary to estimate a population mean to within 1 unit and with 90% confidence, given the std. dev. is 10.

$$n = \left(\frac{z^* \sigma}{m} \right)^2 = \left(\frac{1.64 * 10}{1} \right)^2 = 269$$

- Suppose you pick the sample and the sample mean was calculated as 150. Estimate the population mean with 90% confidence.

$$\begin{aligned} \bar{x} \pm z^* \frac{\sigma}{\sqrt{n}} &= 150 \pm 1.64 \frac{10}{\sqrt{269}} \\ &= 150 \pm 1 \\ &= (149, 151) \end{aligned}$$

Inference when σ is unknown

- In practice, σ is unknown
- Get the one-sample t statistic

with $n - 1$ degrees of freedom

$$t = \frac{\bar{x} - \mu}{s / \sqrt{n}}$$

One sample t procedures

A level C confidence interval for μ

$$\bar{x} \pm t^* \frac{s}{\sqrt{n}}$$

where t^* is the upper $(1-c)/2$ critical value for the $t(n-1)$ distribution

The interval has the form

$$\text{estimate} + t^* \text{SE}_{\text{estimate}}$$

Robustness

- *Sample size less than 15*: Use t procedures only if the data are close to Normal.
- *Sample Size at least 15*: t procedures can be used except in the presence of outliers or strong skewness.
- *Large Samples ($n > 40$)*: t procedures can be used even for clearly skewed distributions.

Tests of significance - assess evidence for a claim about a population

- Null Hypothesis H_0

The statement being tested. Usually a statement of no effect or no difference Ex: Ad has **no effect** on purchase

- Alternative Hypothesis H_a

Claim we are trying to find evidence for Ex: Ad **works**

Example: $H_0 : \mu = 187$

$H_a : \mu > 187$

Test of significance

1. State the *null hypothesis* H_0 and the *alternative hypotheses* H_a .

The test is designed to assess the strength of evidence against H_0 ; H_a is the statement we will accept if we reject H_0

2. Select a significance level.
3. Calculate the *test statistic*.
4. Find the *P-value* for the observed data.
5. State a conclusion.
If $P\text{-value} \leq \alpha$ reject H_0
If $P\text{-value} > \alpha$ accept H_0

Stating hypotheses

- The null hypothesis H_0 is a claim that we will try to find evidence *against*

H_0 is of the form $H_0: \mu = \mu_0$

- The alternative hypothesis H_a is the claim about the population that we are trying to find evidence *for*

The alternative hypothesis can be **one-sided** if we are interested in deviations from the null hypothesis in one direction

H_a is of the form $H_0: \mu > \mu_0$

or $H_0: \mu < \mu_0$

The alternative hypothesis can be two-sided if we are interested in any deviation from the null hypothesis

H_a is of the form $H_a: \mu \neq \mu_0$

Two-sided P -value

When H_a states that μ is simply unequal to μ_0 , values of z away from zero in either direction count against the null hypothesis

The P -value is the probability that a standard normal is at least as far from zero as the observed z .

If the test statistics is $z=1.7$, the P -value is the probability that $Z \leq -1.7$ or $Z \geq 1.7$

$$\begin{aligned} P(Z \leq -1.7 \text{ or } Z \geq 1.7) &= 2P(Z \geq 1.7) \\ &= 2(1 - 0.9554) = 0.0892 \end{aligned}$$

Example

An analytical laboratory is asked to evaluate the claim that the concentration of the active ingredient in a specimen is 0.86%. The lab makes 3 repeated analyses of the specimen. The mean result is 0.8404. The true concentration is the mean μ of the population of all analyses of the specimen. The standard deviation of the analysis process is known to be $\sigma = 0.0068$. Is there significant evidence at the 1% level that $\mu \neq 0.86$?

1. $H_0: \mu = 0.86$
 $H_a: \mu \neq 0.86$
2. Let $\alpha = 0.01$
3. $z = -4.99$
4. $P\text{-value} < .01$
5. Reject H_0 . Results are statistically significant at the 1% level.

Another way

Suppose we calculate a 99% confidence interval for μ

$$\begin{aligned}\bar{x} \pm z^* \frac{\sigma}{\sqrt{n}} &= 0.8404 \pm 2.576 \frac{0.0068}{\sqrt{3}} \\ &= 0.8404 \pm 0.0101 \\ &= 0.8303 \text{ to } 0.8505\end{aligned}$$

Do you *think* that $\mu = 0.86$?

No, because 0.86 isn't in the 99% confidence interval

Confidence intervals

A level α two-sided test of

$$H_0 : \mu = \mu_0 \text{ versus}$$

$$H_a : \mu \neq \mu_0$$

rejects the null hypothesis H_0 when μ_0 falls outside a $C = 1 - \alpha$ confidence interval for μ

Example: The 99% confidence interval for μ in the previous example is (0.83, 0.85)

The hypothesized value $\mu_0 = 0.86$ falls outside this confidence interval, so *reject* H_0 at the 1% significance level