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Introduction to Biostatistics - Lecture 2: Statistical Inference Procedures

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Introduction to Biostatistics

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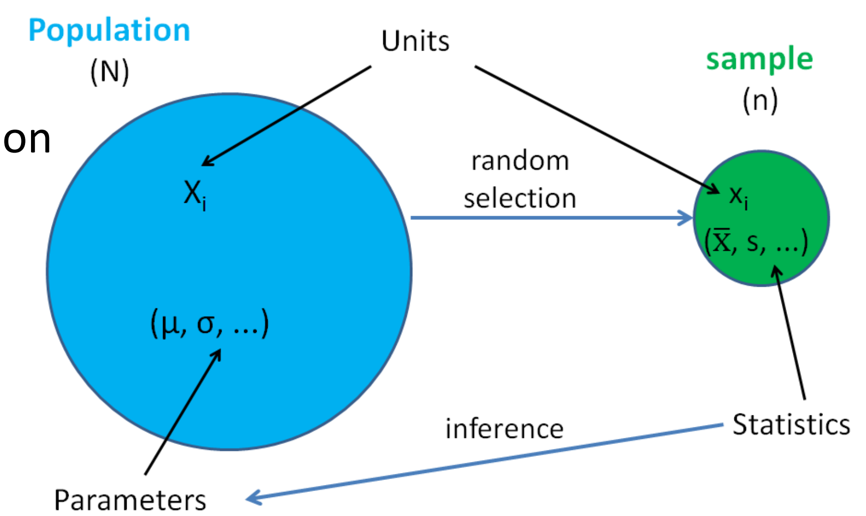
Lecture 2:

- Statistical Inference Procedures
  - Hypothesis test for population average
  - Hypothesis test for comparing means
  - Power and sample size

Statistical Inference

Two broad areas of statistical inference:

- **Estimation:** Use sample statistics to estimate the unknown population parameter.
  - **Point Estimate:** the best single value to describe the unknown parameter.
  - **Standard Error (SE):** standard deviation of the sample statistic. Indicates how precise is the point estimate.
  - **Confidence Interval (CI):** the range with the most probable values for the unknown parameter with a (1-α)% level of confidence.



- **Hypothesis Testing:** Test a specific statement (assumption) about the unknown parameter.

Statistical Inference for population average μ

Estimation: Point Estimate & Standard Error

- Suppose **X** a variable (e.g., systolic BP, hypertension, # of prior complications) from a population of size N with average $\boldsymbol{\mu}$ and standard deviation $\boldsymbol{\sigma}$.
- We select a random sample $x_1, x_2, \ldots, x_n$ of size n
- **Point Estimate** of μ : $\bar{x}$
- **Standard error** of $\bar{x}$: Standard Deviation of all possible $\bar{x}$'s
- From the central limit theorem (CLT), for n large ($n \geq 30$):

$$\bar{x} \sim N(\mu, \frac{\sigma}{\sqrt{n}})$$

- If σ also unknown we can estimate from the sample standard deviation **s**.

The Central Limit Theorem (CLT)

Suppose X from a population (N) with **μ** and **σ**.

- If we take random samples (n) with replacement from the population, for **large "n"** the distribution of the sample mean $\bar{x}$ is approximately normally distributed with $\mu_{\bar{x}} = \mu$ and $\sigma_{\bar{x}} = \frac{\sigma}{\sqrt{n}}$, i.e.:

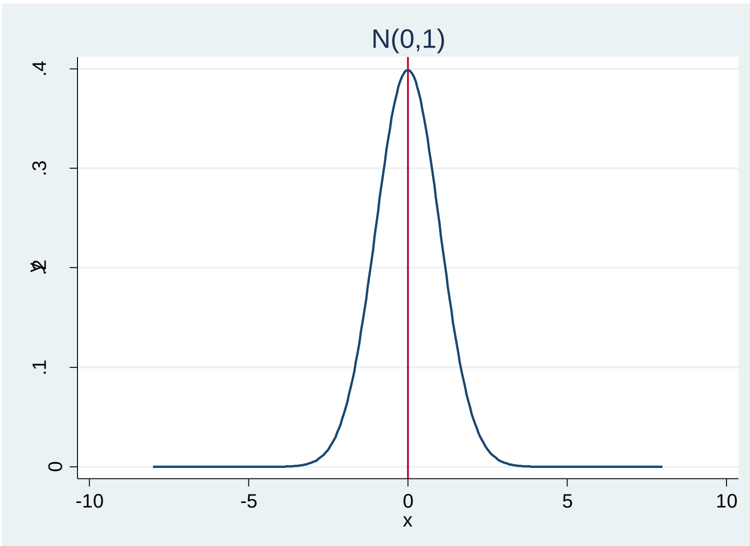
$$\bar{x} \sim N(\mu, \frac{\sigma}{\sqrt{n}})$$

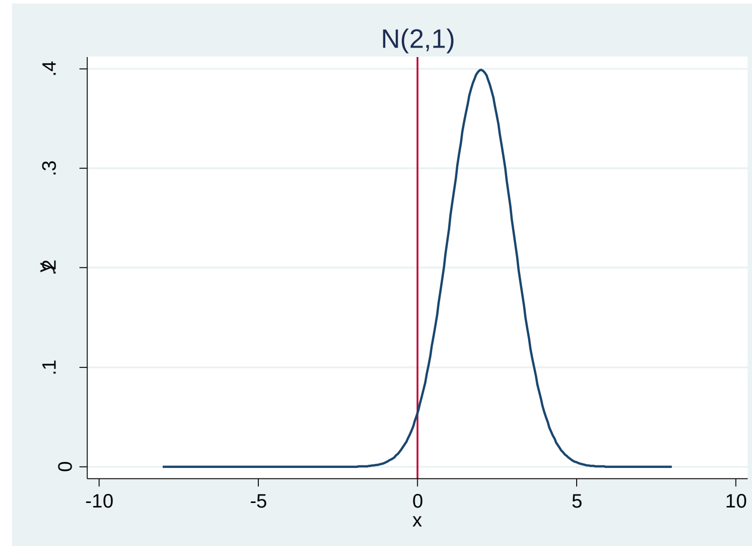
Importance:

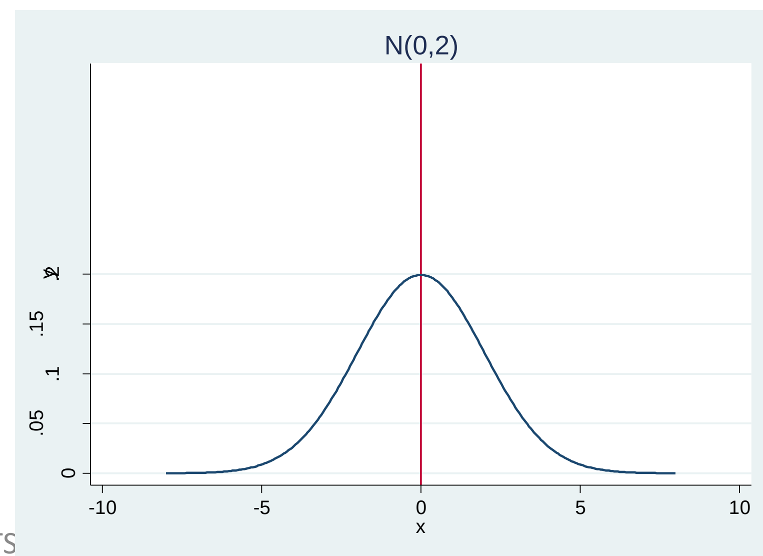
- The distribution of the **sample mean ($\bar{x}$)** is approximately normal even if X does **not** follow N(μ, σ).
- Sample mean is very useful for statistical inference.

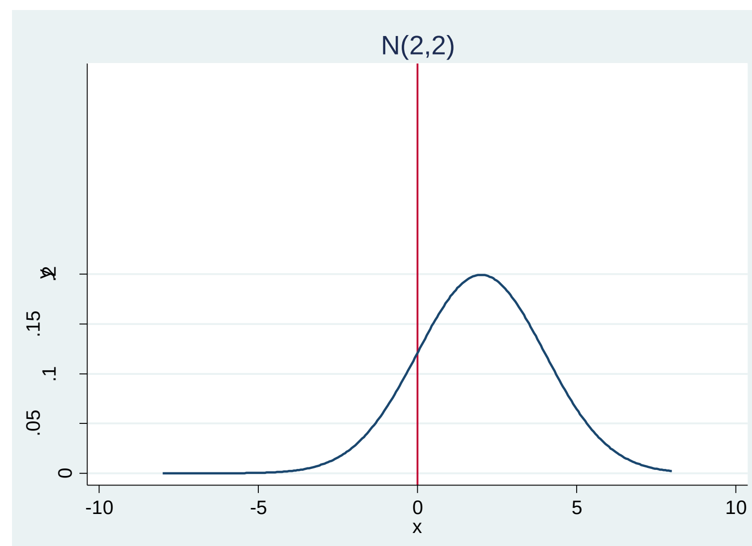
Normal Distribution

Examples:







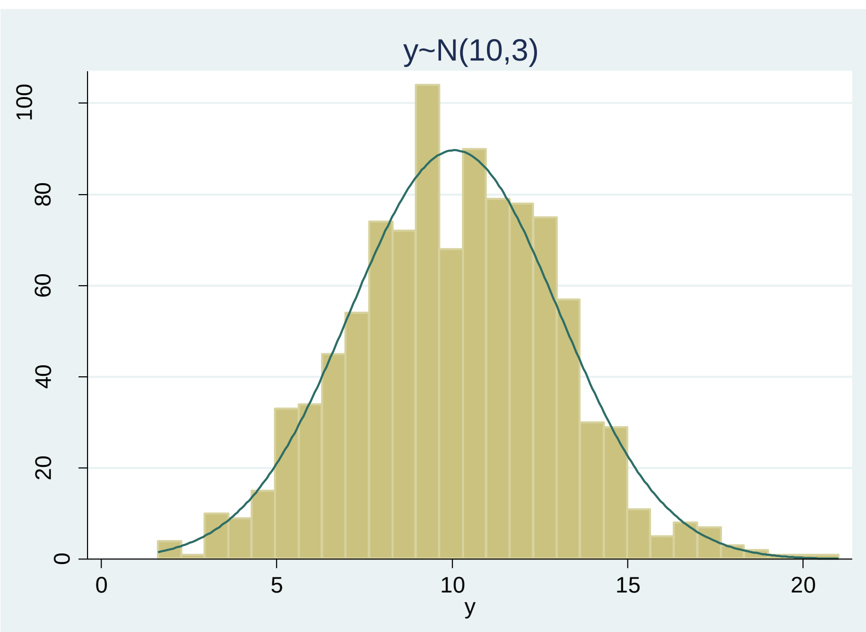


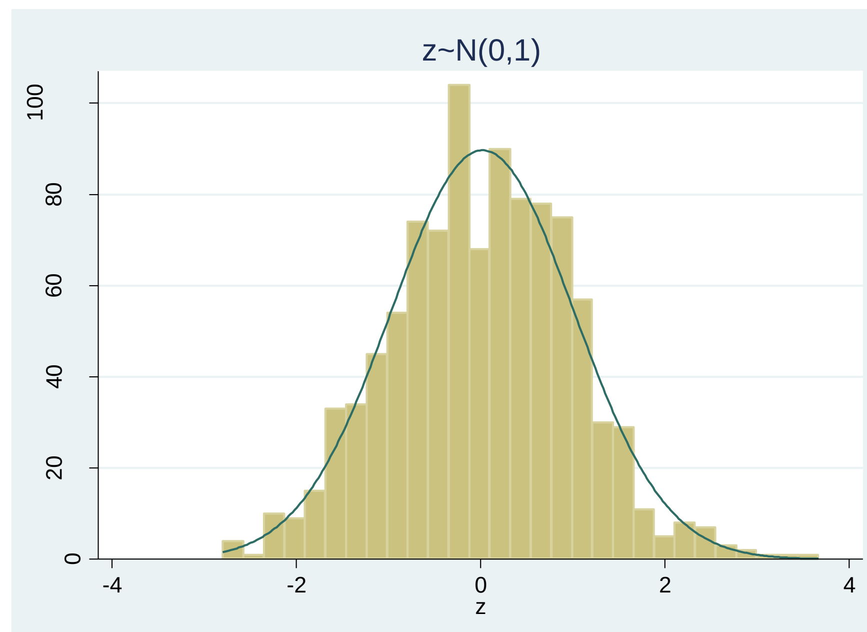
The Standard Normal Distribution

$\bar{x} \sim N(\mu, \sigma/\sqrt{n})$ can be transformed to a $Z \sim N(0, 1)$:

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

- N(0, 1) is called the standard normal distribution
- Z is the standardized value of $\bar{x}$
- Standardized values make comparable variables that are measured in different units, or have different variability





Statistical Inference for population average μ

Estimation: Confidence Interval

- **Confidence Interval (CI)**: a range of values that are likely to cover the true parameter value with a level of confidence (1-α)% assigned to it. The most common choice for α is 5%.

- Usually CIs are symmetric around the point estimate.

- From the central limit theorem (CLT), for n large ($n \geq 30$):

$$\bar{x} \sim N(\mu, \frac{\sigma}{\sqrt{n}})$$

- Hence,

$$Z = \frac{\bar{x} - \mu}{\sigma / \sqrt{n}} \sim N(0, 1)$$

Statistical Inference for population average **μ**

Estimation: Confidence Interval

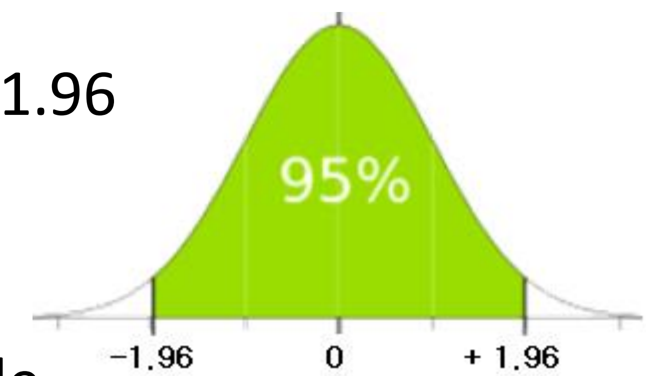
- E.g., (1-α)=95% CI for μ

<u>95% CI for average **μ**</u> : $[\,\bar{x} - 1.96\cdot(\sigma/\sqrt{n})\ ,\ \bar{x} + 1.96\cdot(\sigma/\sqrt{n})]$

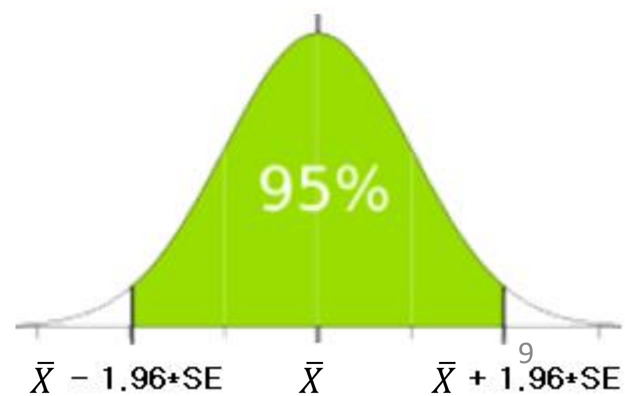
How we derived its 95% CI?

- 95% of Z around 0 is between -1.96 and 1.96

[or $Z_{0.025} = -1.96$ and $Z_{0.975} = 1.96$]



- Remember that Z does not have any scale because it is standardized. We need the scale back to calculate 95% CI.



Statistical Inference for population average μ

Estimation: Confidence Interval

- Based on the percentiles of the N(0,1) there are some commonly reported CIs:

(1-α)% CI	α	α/2	1-α/2	$Z_{\alpha/2}$	$Z_{1-\alpha/2}$
80%	20	10	90	-1.28	1.28
90%	10	5	95	-1.64	1.64
95%	5	2.5	97.5	-1.96	1.96
99%	1	0.5	99.5	-2.58	2.58

Example of CIs: The Framingham Heart Study

- Can you calculate 95% CIs based only on descriptive statistics for the systolic blood pressure?

```
library(psych)
describe(dat1$sysbp)
```

```
> library(psych)
> describe(dat1$sysbp)
   vars     n   mean   sd median trimmed   mad  min max range skew kurtosis   se
X1    1 11627 136.32 22.8    132  134.34 20.76 83.5 295 211.5 0.94     1.37 0.21
```

$$95\%\ \text{CI} : [\ \bar{x} - 1.96 \cdot (\sigma/\sqrt{n})\ ,\ \bar{x} + 1.96 \cdot (\sigma/\sqrt{n})]$$

$$= [136.32 - 1.96 \cdot 0.21\ ,\ 136.32 + 1.96 \cdot 0.21]$$

$$= [135.91,\ 136.73]$$

Example of CIs: The Framingham Heart Study

- Is there any way to calculate 95% CI directly?

```
t.test(dat1$sysbp)
```

```
> t.test(dat1$sysbp)

        One Sample t-test

data:  dat1$sysbp
t = 644.76, df = 11626, p-value < 2.2e-16
alternative hypothesis: true mean is not equal to 0
95 percent confidence interval:
 135.9097 136.7386
sample estimates:
mean of x
 136.3241
```

Hypothesis Testing for the mean **μ**

- Suppose **X** continuous from a population with mean **μ** and standard deviation **σ**.
- What is the value of **μ**?
- We select a random sample from that population and try to make inference about **μ**.

Statistical Inference for population average μ
Key Concepts in Hypothesis Testing

- **Null hypothesis (H_0):**
  - An explicit statement about an unknown parameter the validity of which you wish to test, e.g., $\mu=\mu_0$
- **Alternative hypothesis (H_1):**
  - An alternative statement about the unknown parameter used to compare your null with, e.g.,
    - $\mu \neq \mu_0$ (two-sided test)
    - $\mu < \mu_0$ (one-sided test)
    - $\mu > \mu_0$ (one-sided test)
- **Errors:**
  - Type I : reject H_0 | H_0 is true (crucial)
  - Type II: do not reject H_0 | H_1 is true (moderate)

Statistical Inference for population average μ
Key Concepts in Hypothesis Testing

Think of **Type I** error as the "***presumption of innocence***" according to which "*everyone is presumed innocent until proven guilty*":

"It is better that ten guilty persons escape than that one innocent suffer" from the principle of Blackstone formula:

- H_0 : a person is innocent
- H_1 : a person is guilty

- Without enough evidences, a person is innocent

What about this?

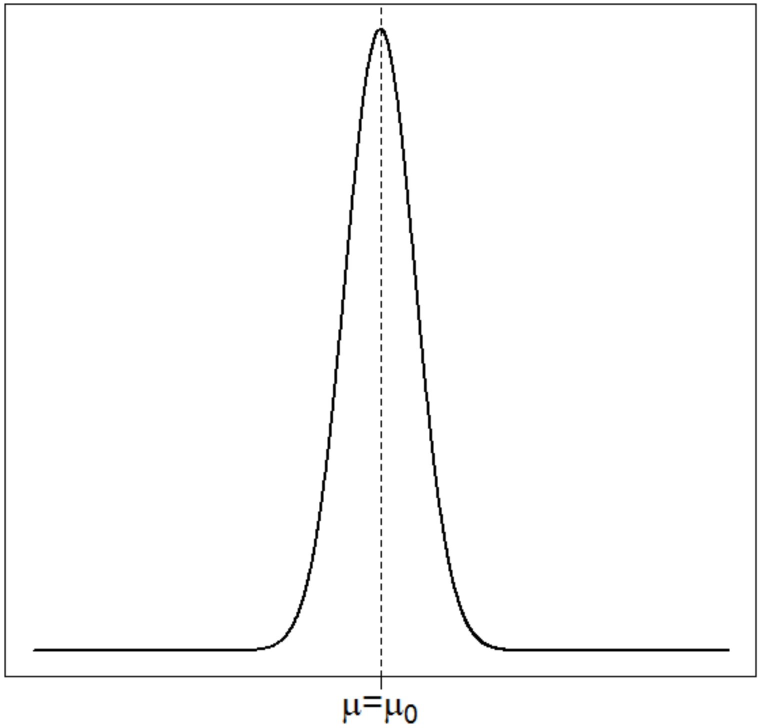
- H_0 : a person is guilty
- H_1 : a person is innocent

- Without enough evidences, a person is guilty

Hypothesis Testing for the mean **μ**

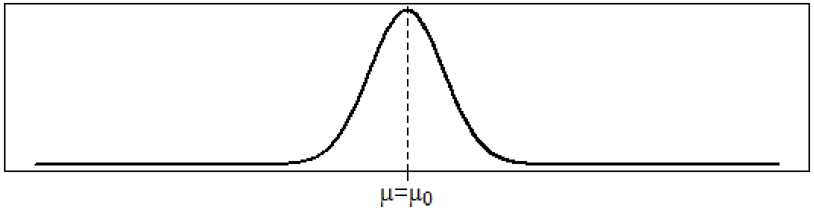
- What is the value of **μ**? (e.g., the population mean of systolic BP is 136.
- Hypothesis Test:

H_0: $\mu = \mu_0 (=136)$



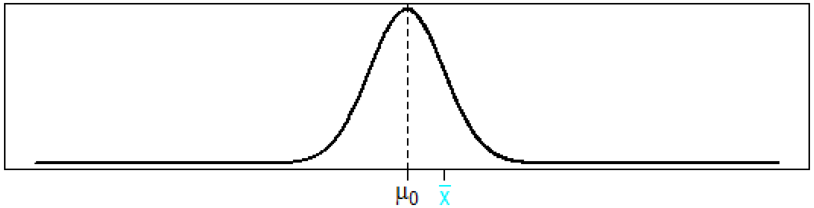
Hypothesis Testing for the mean **μ**

- <u>What is the value of **μ**</u>?



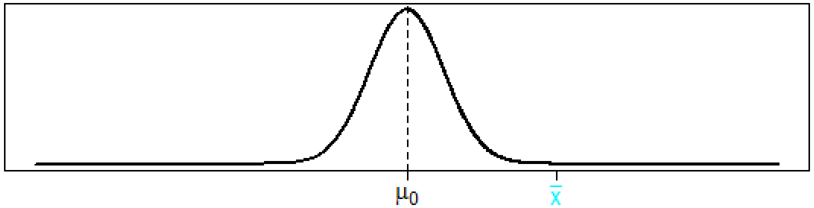
- Hypothesis Test:

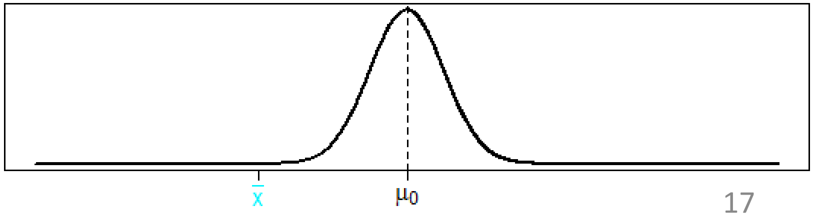
  H_0: $\mu = \mu_0(=136)$



- Random sample:

  $\bar{\mathbf{x}}$





Hypothesis Testing for the mean μ

- What is the value of $\boldsymbol{\mu}$?

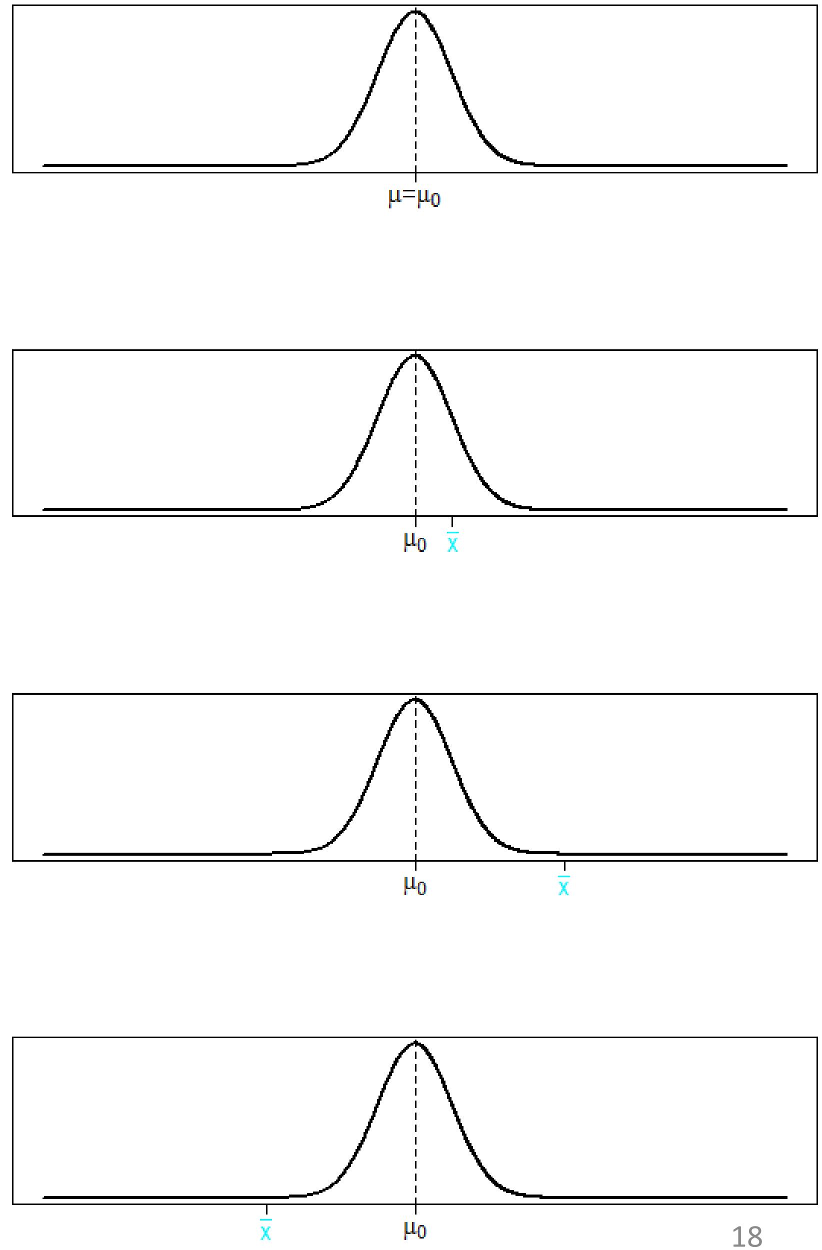
- Hypothesis Test:

  H_0: $\mu = \mu_0$ (?)

- Random sample:

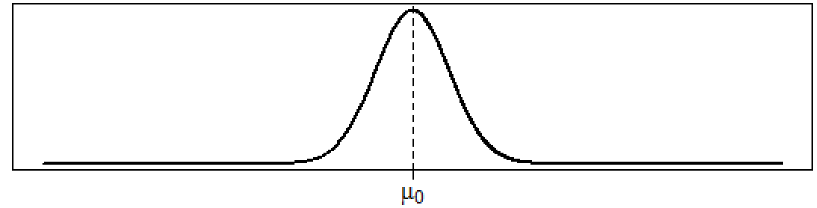
  $\bar{x}$

- If $\bar{x}$ close to $\mu_0 \rightarrow$ H_0 probable
- If $\bar{x}$ far from $\mu_0 \rightarrow$ H_0 not probable



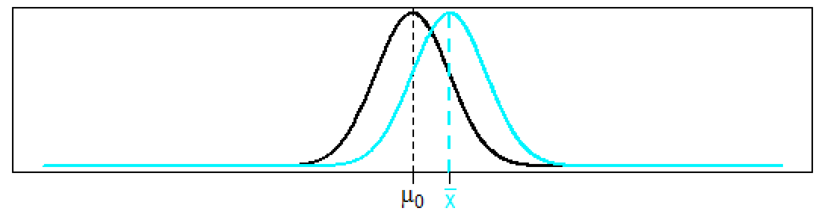
Hypothesis Testing for the mean $\boldsymbol{\mu}$

- <u>What is the value of $\boldsymbol{\mu}$</u>?



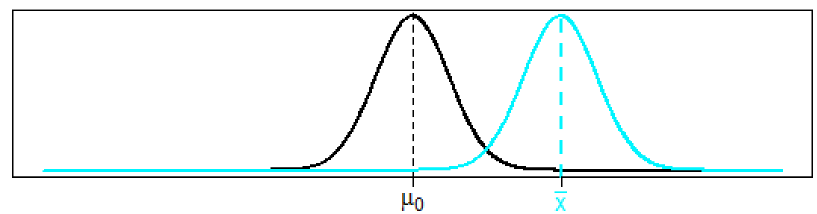
- Hypothesis Test:

  H_0: $\mu = \mu_0$ (?)

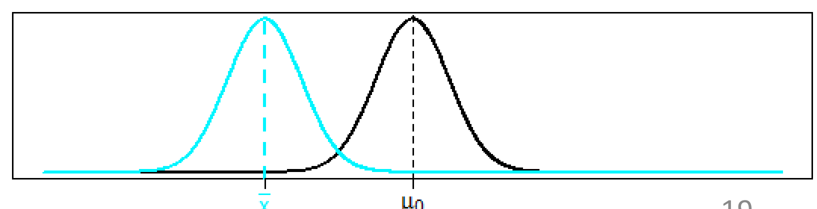


- Random sample:

  $\bar{\mathbf{x}}$



- If $\bar{\mathbf{x}}$ close to $\mu_0 \rightarrow$ H_0 probable
- If $\bar{\mathbf{x}}$ far from $\mu_0 \rightarrow$ H_0 not probable



Statistical Inference for population average μ
Key Concepts in Hypothesis Testing

- **Test Statistic:**
  - A summary measure of your sample, with known distribution under H_0, used for testing the null hypothesis (H_0), e.g.,

Test Statistic

$$Z_0 = \frac{\bar{x} - \mu_0}{\sigma / \sqrt{n}} \overset{H_0}{\sim} N(0, 1)$$

- **Critical points:**
  - Values (percentiles) of the known distribution of the test statistic above or below which the probability of Type I Error is α%, e.g.,

$$Z_{\alpha},\ \ Z_{\alpha/2},\ \ Z_{1-\alpha/2},\ \ t_{1-\alpha/2,\, d.f.}\ ,\ \ \text{etc.}$$

Statistical Inference for population average μ
Hypothesis Test

- Example: Hypothesis testing about the population mean μ, at α% level of significance
- H_0: $\mu = \mu_0$
- H_1: $\mu \neq \mu_0 \quad \Rightarrow \quad \mu = \mu_1 \neq \mu_0$
- CLT $\rightarrow \bar{x} \sim N(\mu, \frac{\sigma}{\sqrt{n}})$ $\quad \Rightarrow \quad Z = \frac{\bar{x}-\mu}{\sigma/\sqrt{n}} \sim N(0, 1)$
- If H_0 is true: $\quad Z_0 = \frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}} \overset{H_0}{\sim} N(0, 1)$
  - Z_0 close to 0 $\quad \rightarrow \quad H_0$ probably true
  - Z_0 "much" different from 0 $\quad \rightarrow \quad H_0$ probably NOT true

Statistical Inference for population average μ
Hypothesis Test

- Example: Hypothesis testing about the population mean μ, at α% level of significance
- $H_0: \mu=\mu_0$
- $H_1: \mu \neq \mu_0 \quad \Rightarrow \quad \mu = \mu_1 \neq \mu_0$
- CLT $\rightarrow \bar{x} \sim N(\mu, \frac{\sigma}{\sqrt{n}}) \qquad \Rightarrow \quad Z = \frac{\bar{x}-\mu}{\sigma/\sqrt{n}} \sim N(0, 1)$
- If H_0 is true: $\quad Z_0 = \frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}} \overset{H_0}{\sim} N(0, 1)$
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How "much"?

Statistical Inference for population average μ
Hypothesis Test

- Example: Hypothesis testing about the population mean μ, at α% level of significance
- H_0: $\mu=\mu_0$
- H_1: $\mu \neq \mu_0 \quad \Rightarrow \quad \mu = \mu_1 \neq \mu_0$
- CLT $\rightarrow \bar{x} \sim N(\mu, \frac{\sigma}{\sqrt{n}}) \quad \Rightarrow \quad Z = \frac{\bar{x}-\mu}{\sigma/\sqrt{n}} \sim N(0, 1)$
- If H_0 is true: $Z_0 = \frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}} \overset{H_0}{\sim} N(0, 1)$
  - Z_0 close to 0 $\rightarrow$ H_0 probably true
  - Z_0 "much" different from 0 $\rightarrow$ H_0 probably NOT true

How "much"? → Critical Z point (Z_c)

Statistical Inference for population average **μ**

Hypothesis Test

- Example: Hypothesis testing about the population mean μ, at α%

$H_0: \mu = \mu_0$

$H_1: \mu \neq \mu_0$

Test statistic: $Z_0 = \frac{\bar{x} - \mu_0}{\sigma / \sqrt{n}}$

Statistical Inference for population average μ

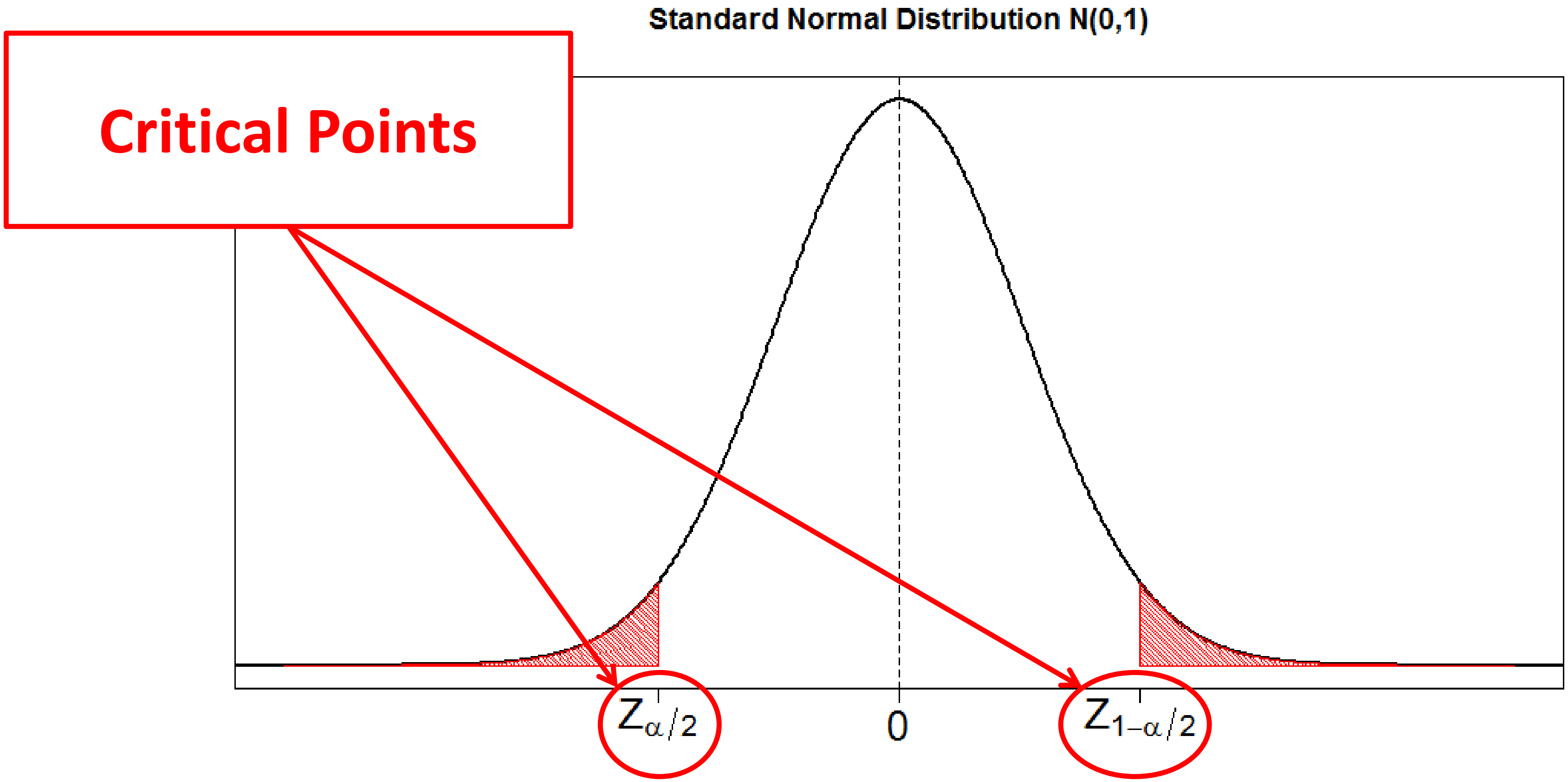
Hypothesis Test

- <u>Example</u>: Hypothesis testing about the population mean μ, at α%

$H_0: \mu = \mu_0$

$H_1: \mu \neq \mu_0$

Test statistic: $Z_0 = \frac{\bar{x} - \mu_0}{\sigma / \sqrt{n}}$



Statistical Inference for population average μ

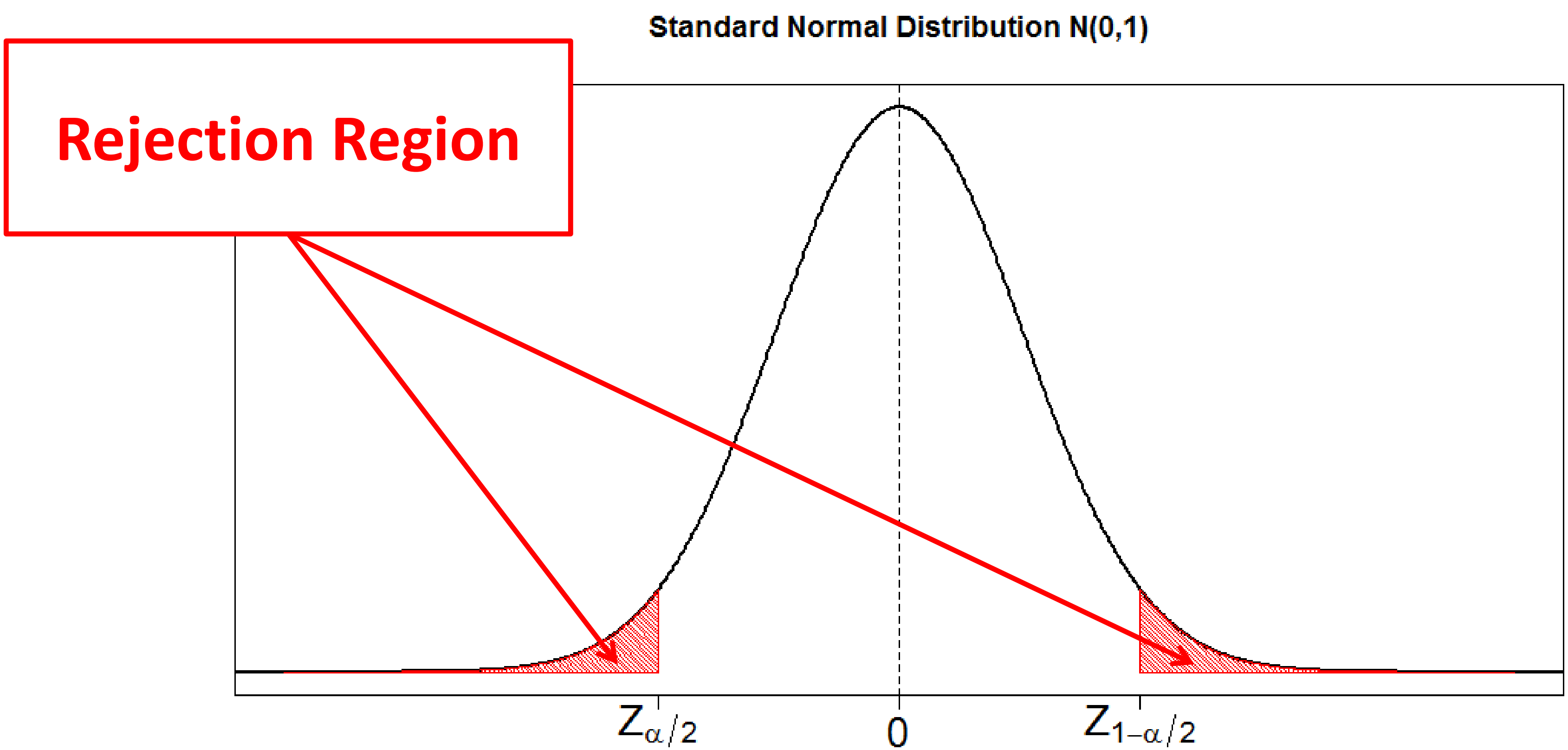
Hypothesis Test

- Example: Hypothesis testing about the population mean μ, at α%

H_0: $\mu = \mu_0$

H_1: $\mu \neq \mu_0$

Test statistic: $Z_0 = \dfrac{\bar{x} - \mu_0}{\sigma / \sqrt{n}}$



Statistical Inference for population average μ

Hypothesis Test

- <u>Example</u>: Hypothesis testing about the population mean μ, at α%

$H_0: \mu=\mu_0$

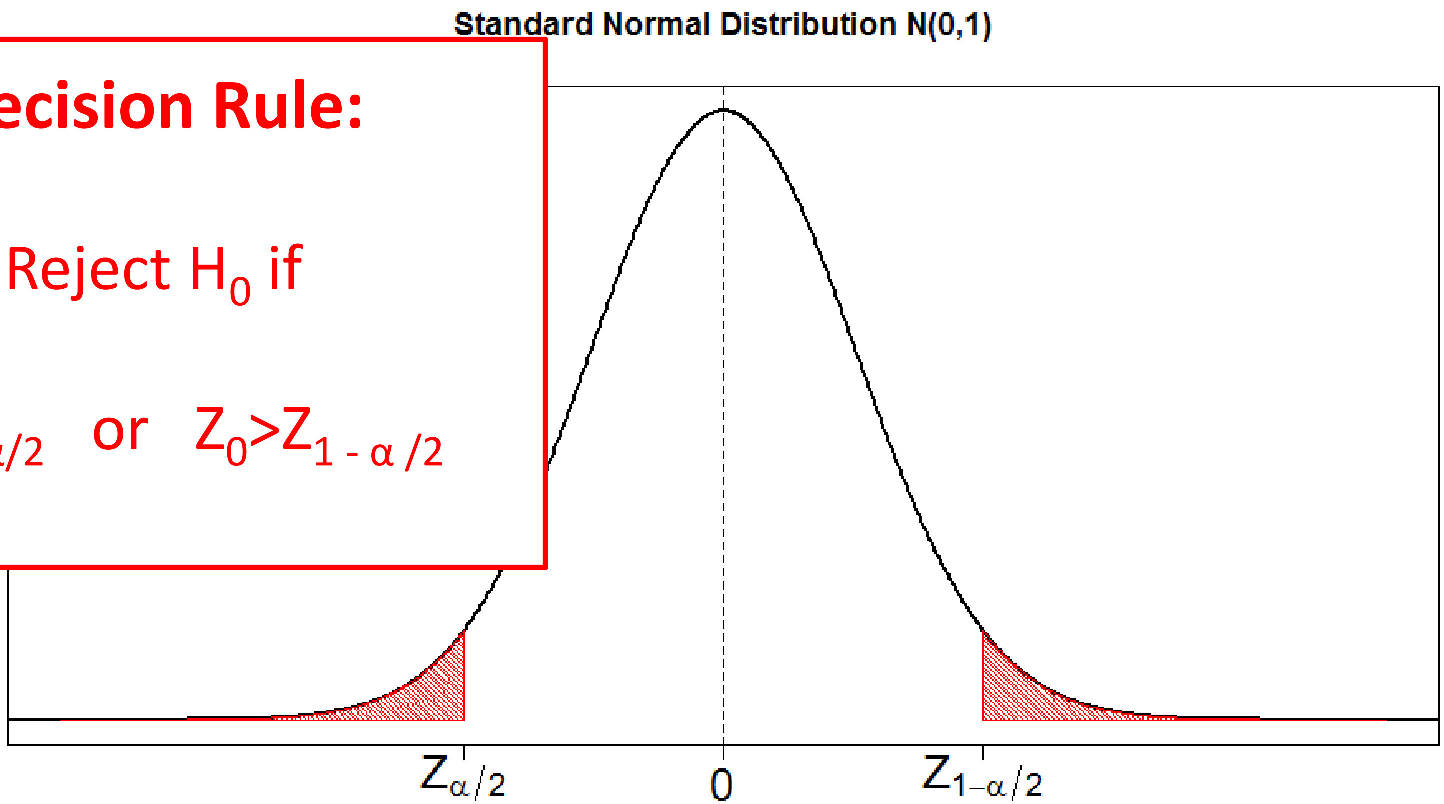
$H_1: \mu \neq \mu_0$

Test statistic: $Z_0 = \frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}}$

Decision Rule:

Reject H_0 if

$Z_0<Z_{\alpha/2}$ or $Z_0>Z_{1-\alpha/2}$



Statistical Inference for population average μ

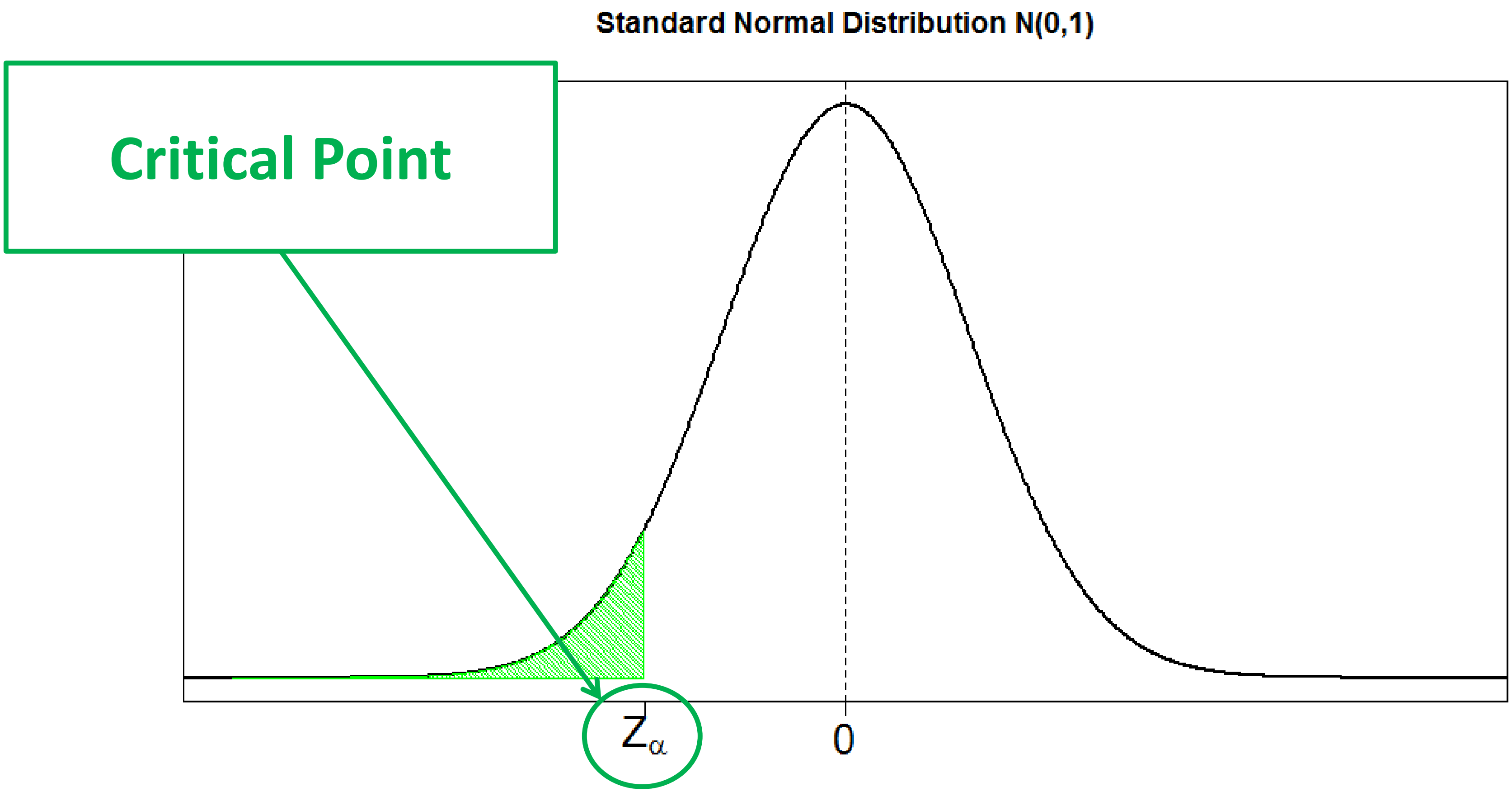
Hypothesis Test

- <u>Example</u>: Hypothesis testing about the population mean μ, at α%

$H_0: \mu=\mu_0$

$H_1: \mu < \mu_0$

Test statistic: $Z_0 = \dfrac{\bar{x}-\mu_0}{\sigma/\sqrt{n}}$



Statistical Inference for population average μ

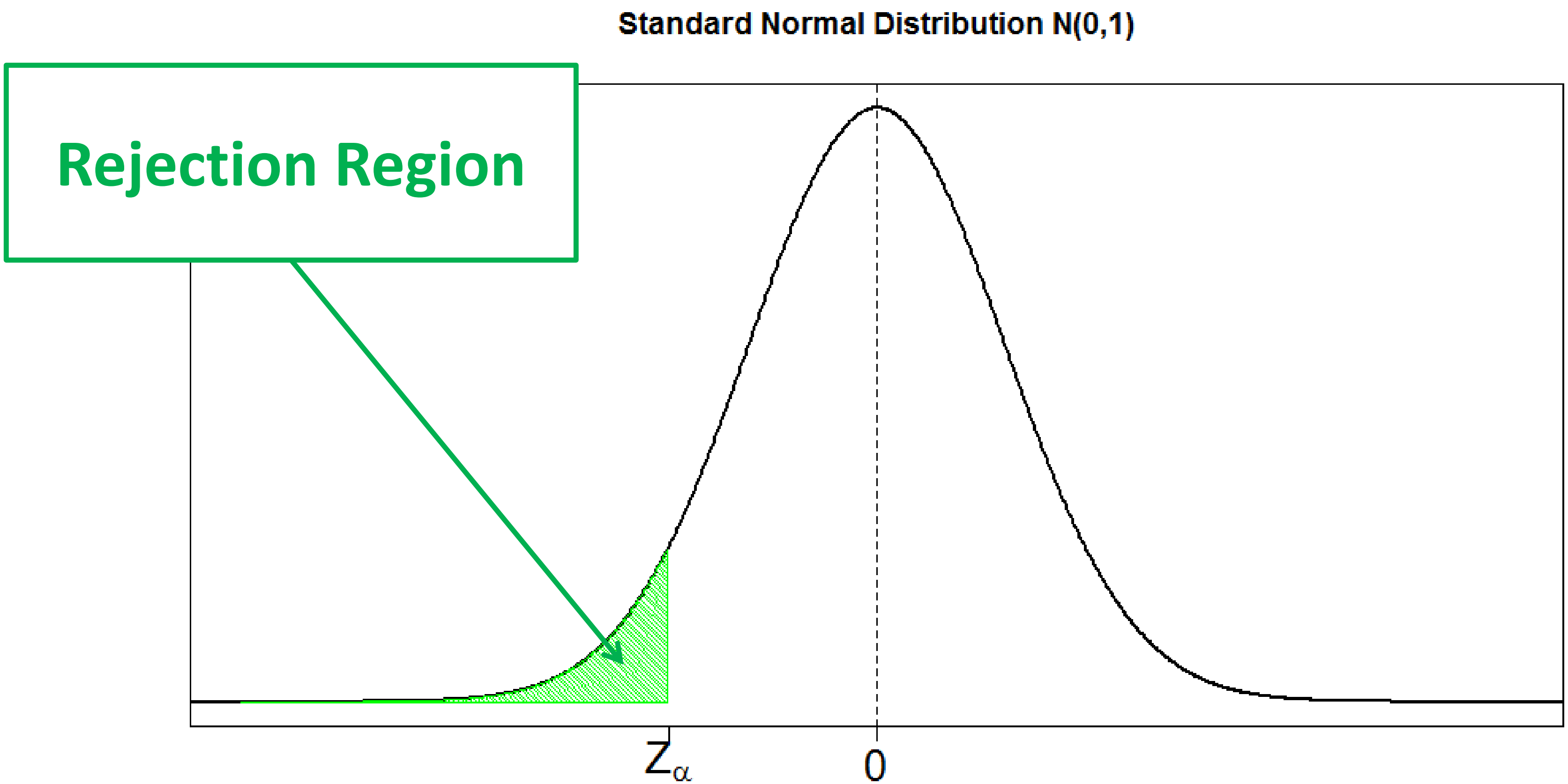
Hypothesis Test

- <u>Example</u>: Hypothesis testing about the population mean μ, at α%

$H_0: \mu=\mu_0$

$H_1: \mu < \mu_0$

Test statistic: $Z_0 = \frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}}$



Statistical Inference for population average μ
Hypothesis Test

- <u>Example</u>: Hypothesis testing about the population mean μ, at α%

$H_0: \mu=\mu_0$

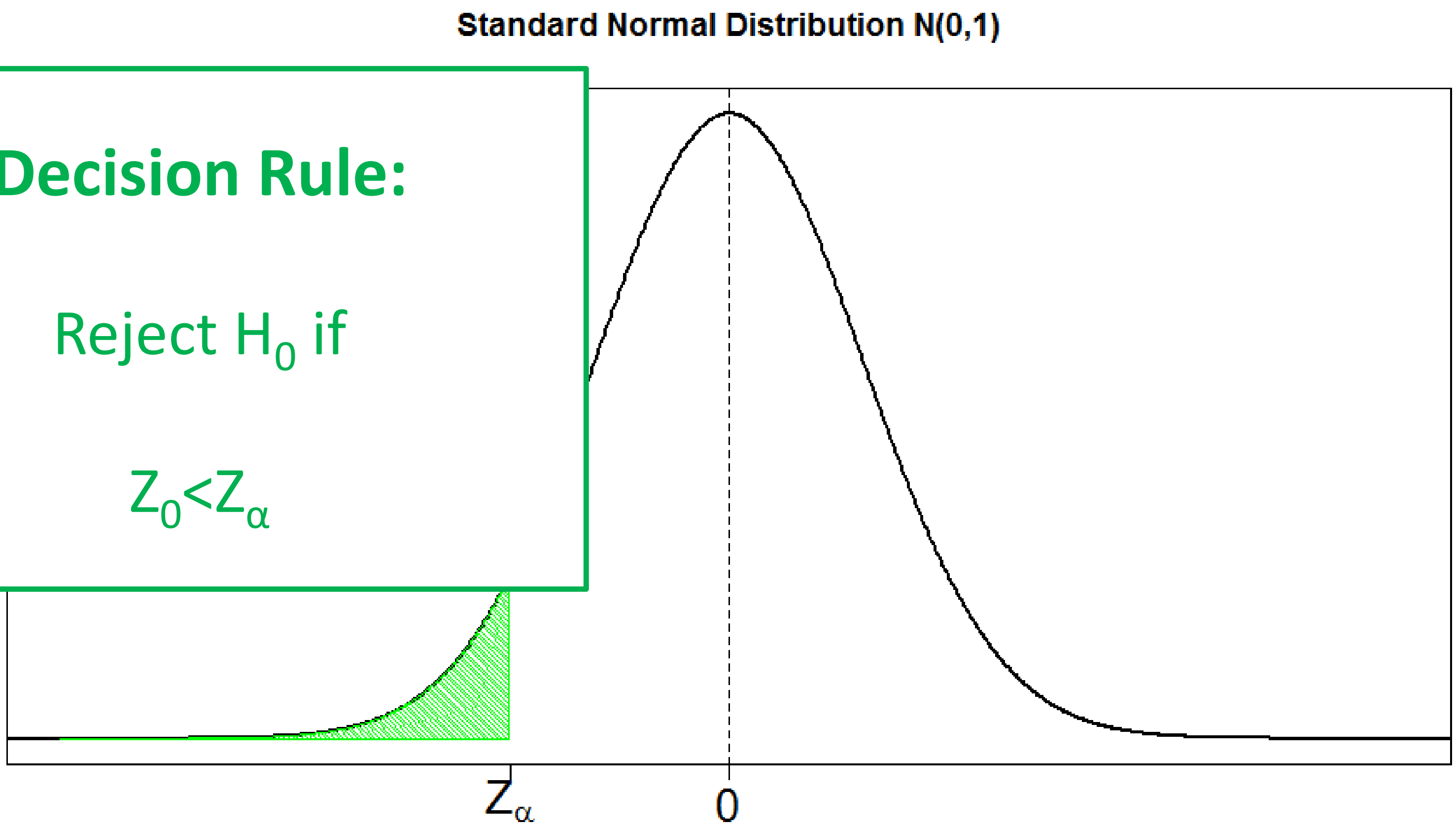
$H_1: \mu < \mu_0$

Test statistic: $Z_0 = \frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}}$

Decision Rule:

Reject H_0 if

$Z_0<Z_\alpha$



Statistical Inference for population average μ

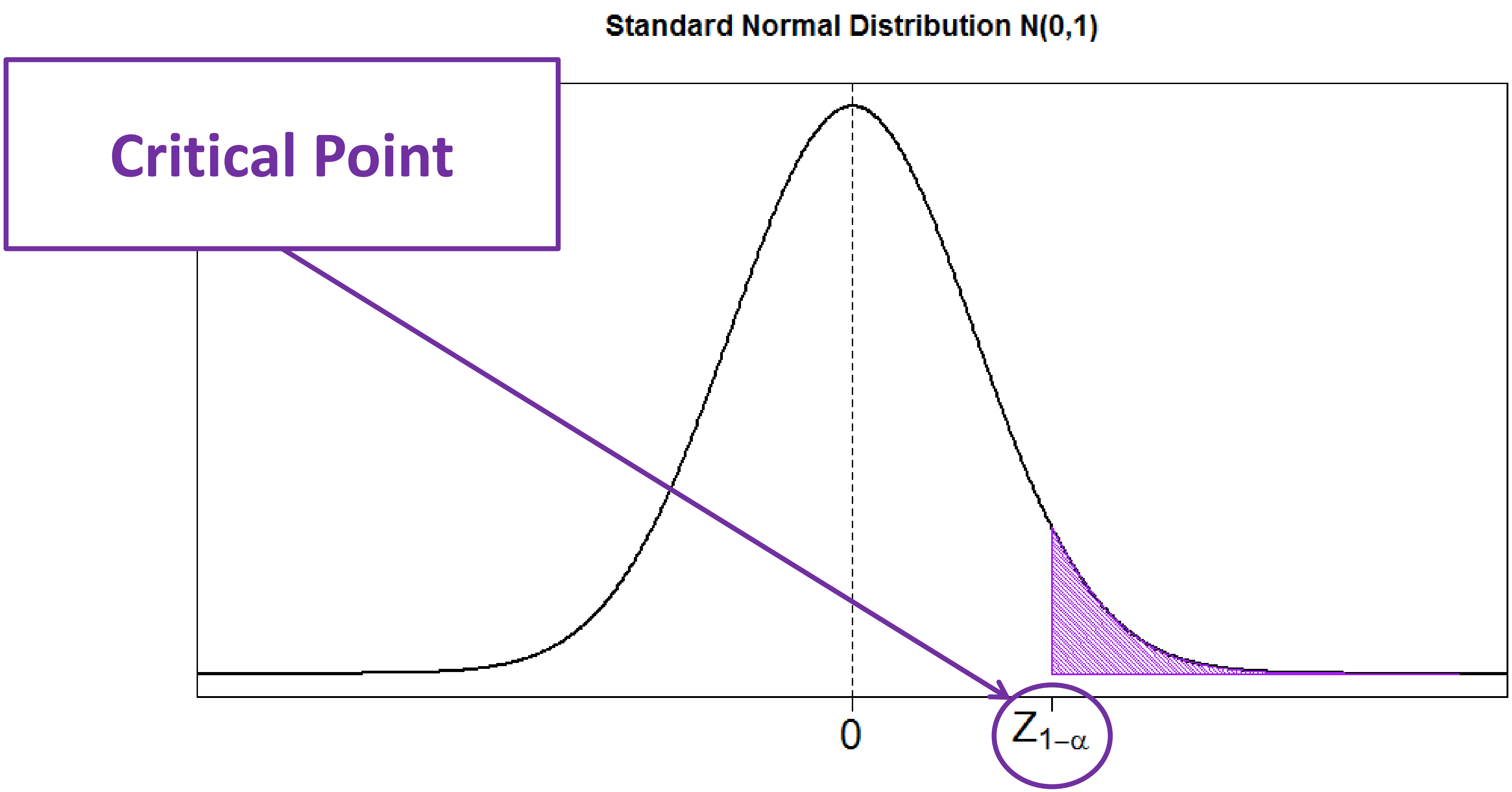
Hypothesis Test

- Example: Hypothesis testing about the population mean μ, at α%

$H_0: \mu = \mu_0$

$H_1: \mu > \mu_0$

Test statistic: $Z_0 = \frac{\bar{x} - \mu_0}{\sigma / \sqrt{n}}$



Statistical Inference for population average μ

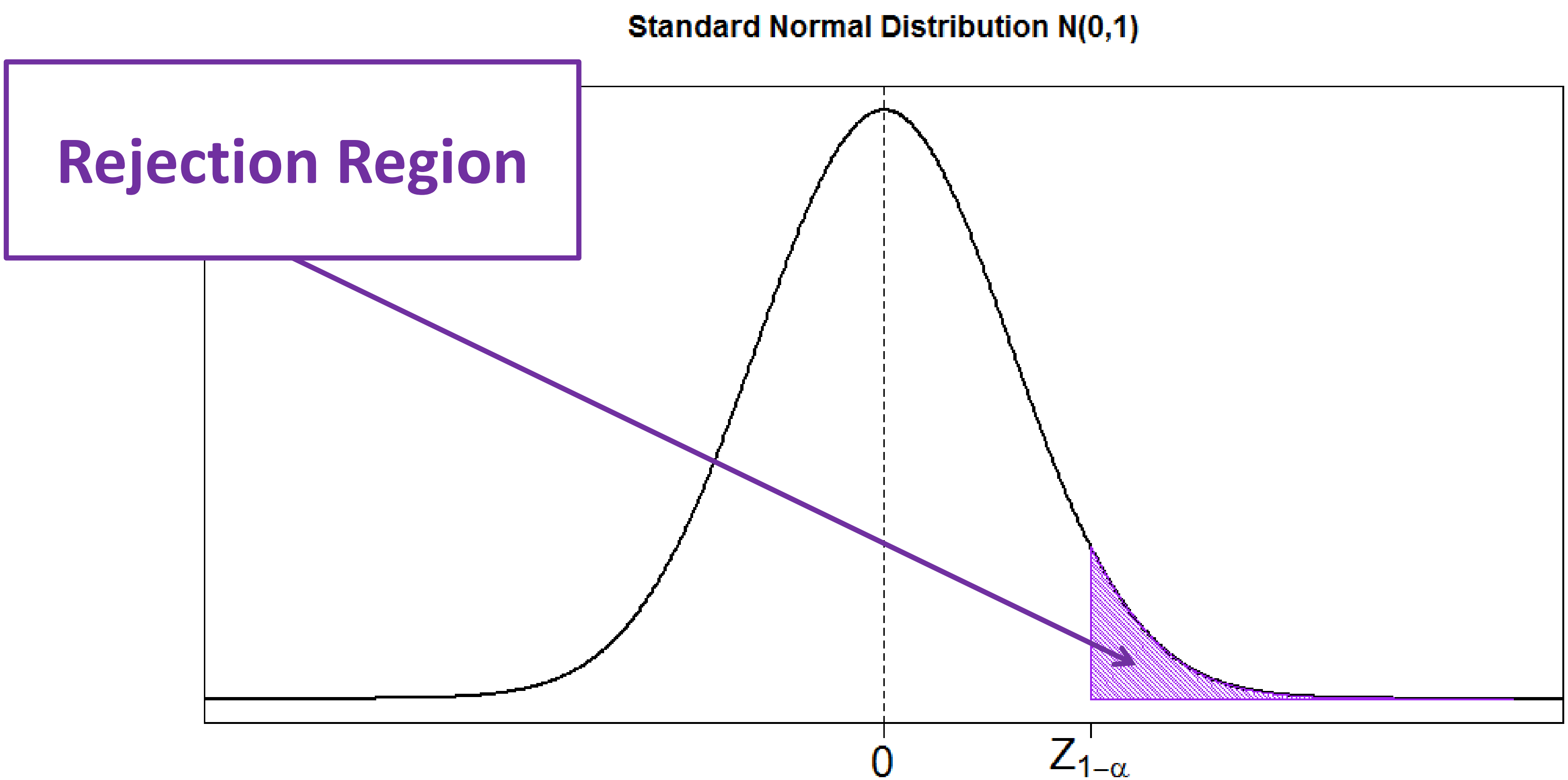
Hypothesis Test

- Example: Hypothesis testing about the population mean μ, at α%

$H_0: \mu = \mu_0$

$H_1: \mu > \mu_0$

Test statistic: $Z_0 = \dfrac{\bar{x} - \mu_0}{\sigma / \sqrt{n}}$



Statistical Inference for population average μ
Hypothesis Test

- Example: Hypothesis testing about the population mean μ, at α%

$H_0: \mu=\mu_0$

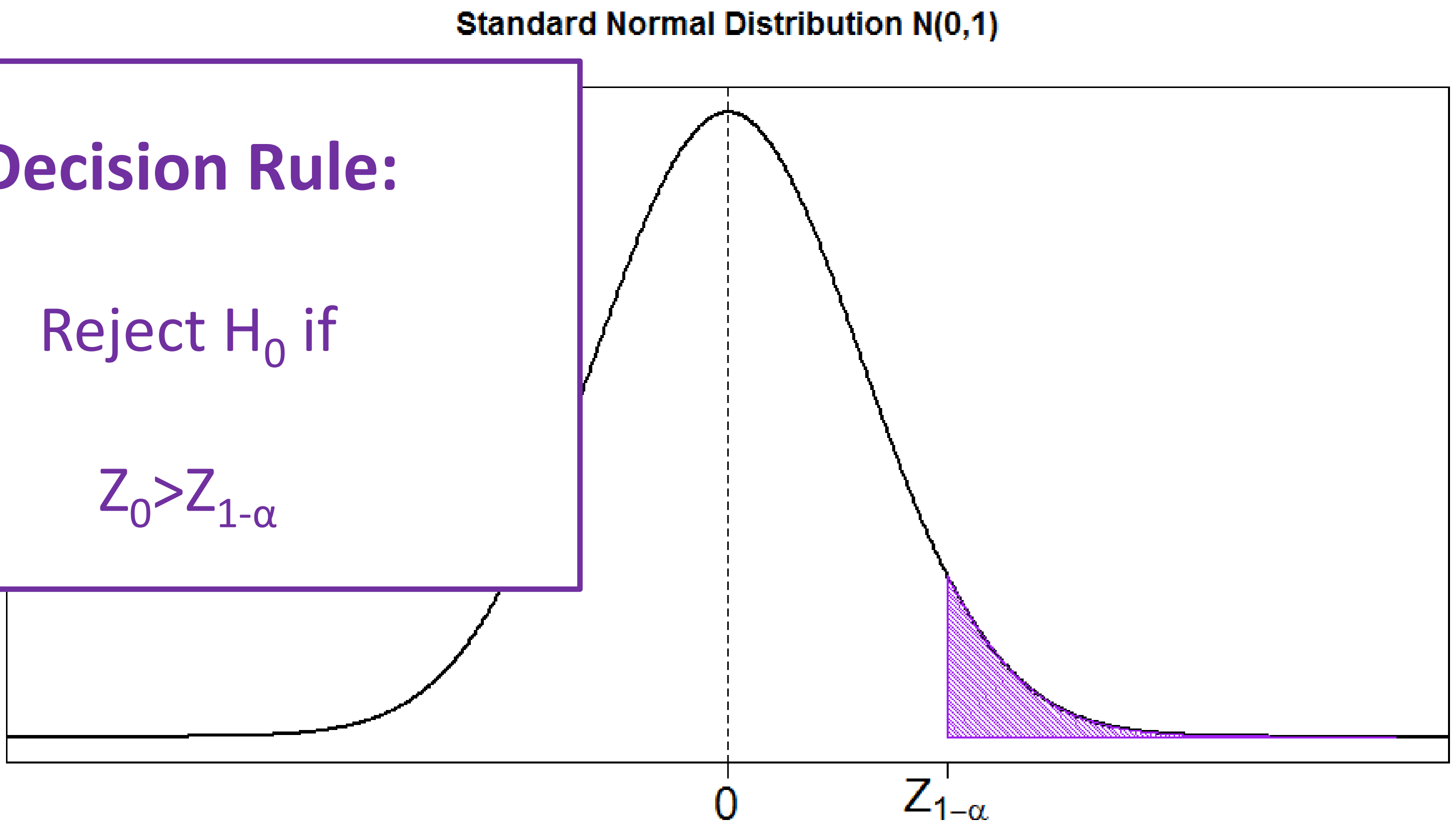
$H_1: \mu > \mu_0$

Test statistic: $Z_0 = \frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}}$

Decision Rule:

Reject H_0 if

$Z_0 > Z_{1-\alpha}$



Statistical Inference for population average μ
Key Concepts in Hypothesis Testing

- **Decision Rule:**
  - What values of the test statistic would indicate the H_0 is probably not supported by the observed data, hence it should be rejected.

- **P-value:**
  - The exact level of significance, i.e., the probability of observing a value as extreme or more extreme than the calculated test statistic under the null hypothesis H_0, e.g.,

$$\text{p-value} = P(Z > Z_0)$$

Statistical Inference for population average μ

Steps in Hypothesis Testing

1. **Set the null hypothesis H_0 and alternative hypothesis H_1**
2. **Set a level of significance α%.**
3. **Calculate a test statistic**
4. **decision rule or**
5. **P-value of the test statistic (preferred)**
6. **conclusion**

Statistical Inference for population average μ

- We will cover examples for three cases
  - 1) Single population: one sample t-test
    - Interested in the population mean
  - 2) Two independent population: two sample t-test
    - Interested in comparing two population means
  - 3) Two dependent population: Paired t test
    - Interested in comparing mean changes within subjects (before vs. after)

Statistical Inference for population average μ
Case 1: One-Sample: two-sided hypothesis Test

- Example: We want to test the following hypothesis about the population mean μ of the systolic blood pressure of the Framingham Heart Study population, at α=5% level of significance:

$$H_0: \mu=130 \quad \text{vs} \quad H_1: \mu \neq 130$$

- Test statistic: $Z_0 = \frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}} = \frac{\bar{x}-\mu_0}{s/\sqrt{n}} = \frac{136.32-130}{22.8/\sqrt{11627}} = 29.91$
- Conclusion: $Z_0 = 29.91 \Rightarrow$ reject H_0 if $|Z_0| > 1.96$
- p-value = $P(Z>|Z_0|) = P(Z<-Z_0) + P(Z > Z_0) = 2*P(Z>29.91) < 0.0001$

```
t.test(dat1$sysbp, mu = 130)

> t.test(dat1$sysbp, mu = 130)

        One Sample t-test

data:  dat1$sysbp
t = 29.911, df = 11626, p-value < 2.2e-16
alternative hypothesis: true mean is not equal to 130
95 percent confidence interval:
 135.9097 136.7386
sample estimates:
mean of x
 136.3241
```

Statistical Inference for population average μ
One-sided hypothesis Test

- Example: We want to test the following hypothesis about the population mean μ of the systolic blood pressure of the Framingham Heart Study population, at α=5% level of significance:

$$H_0: \mu=130 \quad \text{vs} \quad H_1: \mu > 130$$

- Test statistic: $Z_0 = \frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}} = \frac{\bar{x}-\mu_0}{s/\sqrt{n}} = \frac{136.32-130}{22.8/\sqrt{11627}} = 29.91$
- Conclusion: $Z_0 = 29.91 \Rightarrow$ reject H_0: if $Z_0 > 1.68$
- p-value = $P(Z > Z_0) = P(Z>29.91) < 0.0001$

```
t.test(dat1$sysbp, mu=130, alternative="greater") ## one-sided H1: mu > 130
```

```
> t.test(dat1$sysbp, mu=130, alternative="greater") ## one-sided H1: mu > 130

        One Sample t-test

data:  dat1$sysbp
t = 29.911, df = 11626, p-value < 2.2e-16
alternative hypothesis: true mean is greater than 130
95 percent confidence interval:
 135.9763      Inf
sample estimates:
mean of x 
 136.3241 
```

Statistical Inference for population average μ
One-sided hypothesis Test

- Example: We want to test the following hypothesis about the population mean μ of the systolic blood pressure of the Framingham Heart Study population, at α=5% level of significance:

$$H_0: \mu=130 \quad \text{vs} \quad H_1: \mu < 130$$

- Test statistic: $Z_0 = \frac{\bar{x}-\mu_0}{\sigma/\sqrt{n}} = \frac{\bar{x}-\mu_0}{s/\sqrt{n}} = \frac{136.32-130}{22.8/\sqrt{11627}} = 29.91$
- Conclusion: $Z_0 = 29.91 \Rightarrow$ reject H_0: if $Z_0 < -1.68$
- p-value = $P(Z < Z_0) = 1$

```
t.test(dat1$sysbp, mu=130, alternative="less") ## one-sided H1: mu < 130
> t.test(dat1$sysbp, mu=130, alternative="less") ## one-sided H1: mu < 130

        One Sample t-test

data:  dat1$sysbp
t = 29.911, df = 11626, p-value = 1
alternative hypothesis: true mean is less than 130
95 percent confidence interval:
     -Inf 136.6719
sample estimates:
mean of x
 136.3241
```

Two Independent Samples

- **Case 2:** two-independent populations (two-samples)
- $\mathbf{X_1}$ 'sysbp' of people **without previous CHD**, with $\boldsymbol{\mu_1}$ and unknown $\boldsymbol{\sigma_1}$
- $\mathbf{X_2}$ 'sysbp' of people **with previous CHD**, with $\boldsymbol{\mu_2}$ and unknown $\boldsymbol{\sigma_2}$

Hypothesis Testing for μ_1-μ_2

- Null hypothesis (H_0): $\mu_1-\mu_2=0 \implies \mu_1=\mu_2$
- Alternative hypothesis (H_1):
  - $\mu_1-\mu_2 \neq 0 \implies \mu_1 \neq \mu_2$ (two-sided test), or
  - $\mu_1-\mu_2 < 0 \implies \mu_1 < \mu_2$ (one-sided test), or
  - $\mu_1-\mu_2 > 0 \implies \mu_1 > \mu_2$ (one-sided test)

Two Independent Samples

- **Case 2:** two-independent populations (two-samples)
  - **Case 2.A: Known** variances
- $\mathbf{X_1}$ 'sysbp' of people **without previous CHD**, with $\boldsymbol{\mu_1}$ and known $\boldsymbol{\sigma_1}$
- $\mathbf{X_2}$ 'sysbp' of people **with previous CHD**, with $\boldsymbol{\mu_2}$ and known $\boldsymbol{\sigma_2}$

Hypothesis Testing for μ_1-μ_2

- Test statistic: $$Z_0 = \frac{\bar{x}_1 - \bar{x}_2}{\sqrt{\frac{s_1^2}{n_1} + \frac{s_2^2}{n_2}}} \overset{H_0}{\sim} N(0, 1)$$
- Decision Rules by H_1: Testing H_0: μ_1-μ_2=0 vs :

H_1	Reject H_0 if:
$\mu_1-\mu_2 \neq 0$	$Z_0 < Z_{\alpha/2}$ or $Z_0 > Z_{1-\alpha/2}$
$\mu_1-\mu_2 < 0$	$Z_0 < Z_{\alpha}$
$\mu_1-\mu_2 > 0$	$Z_0 > Z_{1-\alpha}$

Two Independent Samples

- **Case 2:** two-independent populations (two-samples)
- X_1 'sysbp' of people **without prevchd**, with μ_1 and unknown σ_1
- X_2 'sysbp' of people **with prevchd**, with μ_2 and unknown σ_2

Hypothesis Testing for μ_1-μ_2

H_0: $\mu_1 = \mu_2$ vs. H_1: $\mu_1 \neq \mu_2$

```
t.test(sysbp ~ prevchd, data=dat1) ## var.equal = FALSE
```

```
> t.test(sysbp ~ prevchd, data=dat1) ## var.equal = FALSE

        Welch Two Sample t-test

data:  sysbp by prevchd
t = -13.036, df = 945.08, p-value < 2.2e-16
alternative hypothesis: true difference in means is not equal to 0
95 percent confidence interval:
 -13.54697 -10.00183
sample estimates:
mean in group 0 mean in group 1
       135.4714        147.2458
```

Two Dependent Samples

- **Case 3:** two-dependent populations (two-samples)
- $\mathbf{X_1}$ 'sysbp' of people **at baseline**, with $\boldsymbol{\mu_1}$ and unknown $\boldsymbol{\sigma_1}$
- $\mathbf{X_2}$ 'sysbp' of people **6yrs after baseline**, with $\boldsymbol{\mu_2}$ and unknown $\boldsymbol{\sigma_2}$
- Suppose variable: $d=x_1-x_2$ from population with $\boldsymbol{\mu_d}$ and $\boldsymbol{\sigma_d}$

Hypothesis Testing for μ_d

- Null hypothesis (H_0): $\mu_d=0$
- Alternative hypothesis (H_1):
  - $\mu_d \neq 0$ (two-sided test), or
  - $\mu_d < 0$ (one-sided test), or
  - $\mu_d > 0$ (one-sided test)

Looks familiar? This is then same as one-sample t-test!

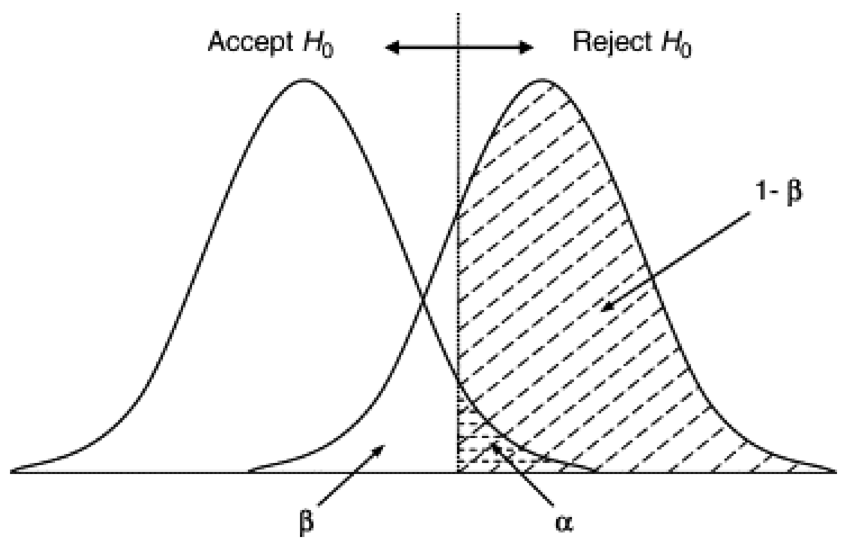
Power and Sample Size Determination

Power = 1- P(Type II error) = 1 - P(do not reject H_0 | H_1 is true) = 1 – β

= P(reject H_0 | H_1 is true)

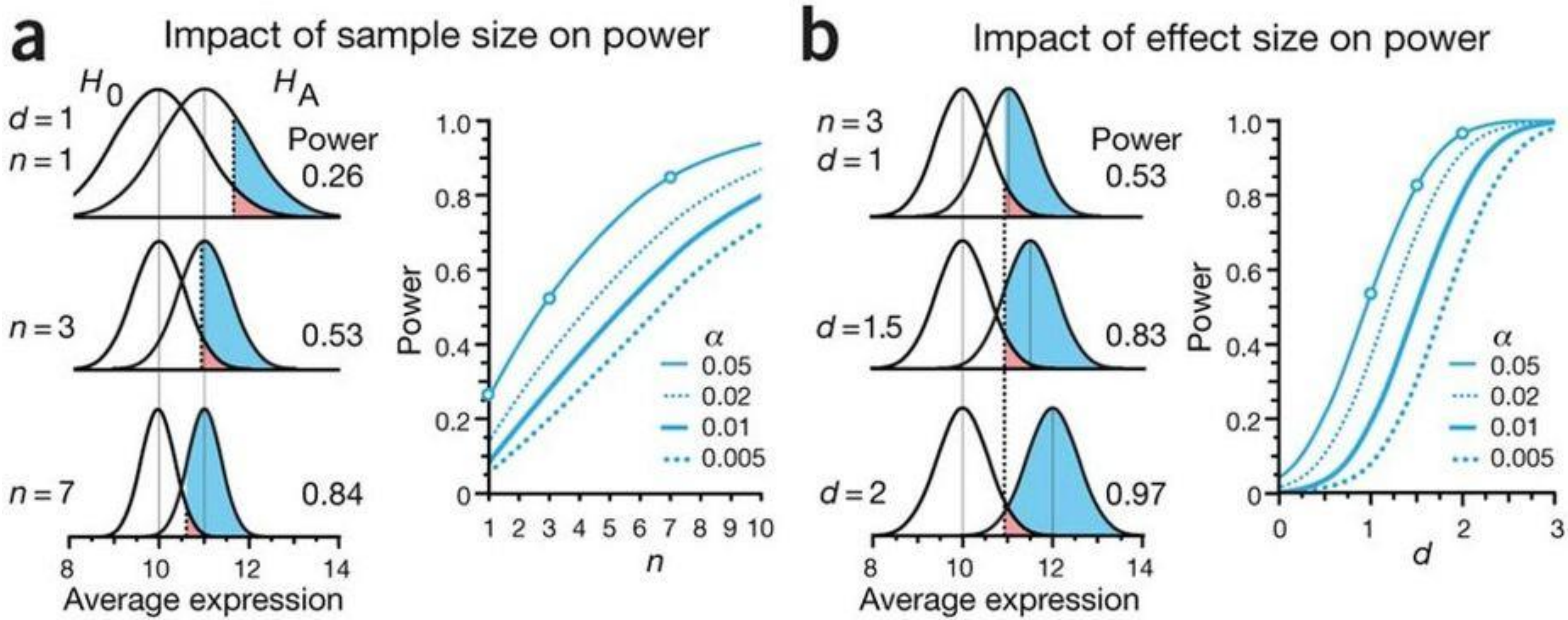
- E.g., the hypothesis: H_0: $\mu=\mu_0$ vs H_1: $\mu = \mu_1 > \mu_0$
- The power of this test is:

Power = P(reject H_0 | H_1 is true) = P($Z_0 > Z_{1-\alpha}$ | $\mu = \mu_1 > \mu_0$)



Power and Sample Size Determination

Power is a function of 1) standard deviation (σ),
2) sample size (n),
2) mean difference (or effect size),
3) type I error (α).



Reference: Krzywinski and Altman, “Power and sample size”, Nature Methods 10, 1139-1140 (2013).

Power and Sample Size Determination

- The power of the test is:

$$\text{Power} = P(\text{reject } H_0 \mid H_1 \text{ is true}) = P\left(Z_1 > Z_{1-\alpha} - \frac{\mu_1 - \mu_0}{\sigma/\sqrt{n}}\right) \qquad (2)$$

- The power of the test depends on:
  - n (**standard deviation**)

    $\sigma \uparrow \Rightarrow$ Power $\downarrow$

  - n (**sample size**)

    $n \uparrow \Rightarrow$ Power $\uparrow$

  - α (**significance level**)

    $\alpha \downarrow \Rightarrow$ Power $\downarrow$

  - $\mu_1 - \mu_0$ (**Effect Size**)

    ES $\uparrow \Rightarrow$ Power $\uparrow$

Sample Size Determination

Case 1: Single population (one-sample):

$$H_0: \mu=100 \quad \text{vs} \quad H_1: \mu\neq100$$

- at α=5% level of significance.
- We want a powerful test with power 80% power.
- The test will reject the null hypothesis if the true mean is 5 units different from 100 (either smaller or larger – two-sided test). Namely, $|\mu-\mu_0|=5$.
- Suppose we know that standard deviation of the outcome variable σ=9.5
- What is the required sample size?

Sample Size Determination

Case 1: single population (one-sample)

H_0: μ=100 vs **H_1: μ≠100** (two-sided test)

```
library(pwr)
pwr.t.test(d = 5/9.5, sig.level=0.05, power = 0.8, type="one.sample")
```

```
> pwr.t.test(d = 5/9.5, sig.level=0.05, power = 0.8, type="one.sample")

     One-sample t test power calculation

              n = 30.3112
              d = 0.5263158
      sig.level = 0.05
          power = 0.8
    alternative = two.sided
```

The total N = 31

Sample Size Determination

Case 2: two dependent populations (two-samples) with unknown variance of the differences

Example: Suppose s_d=7. We want to test the hypothesis:

$$H_0: \mu_1=\mu_2=100 \quad \text{vs} \quad H_1: \mu_1 \neq \mu_2$$

- at α=5% level of significance.
- We want to detect $|\mu_1-\mu_2|=5$.
- With power=80%

What is the required sample size?

Sample Size Determination

Case 2: two dependent populations (two-samples)

$H_0: \mu_1=\mu_2=100$ vs $\mathbf{H_1: \mu_1 \neq \mu_2}$ (two-sided test)

<u>Assume</u>:

$\rightarrow$ unknown variance of the differences,

i.e., $s_d=7$

```
pwr.t.test(d = 5/7, sig.level=0.05, power = 0.8, type="two.sample")
```

```
> pwr.t.test(d = 5/7, sig.level=0.05, power = 0.8, type="two.sample")

     Two-sample t test power calculation 

              n = 31.75708
              d = 0.7142857
      sig.level = 0.05
          power = 0.8
    alternative = two.sided

NOTE: n is number in *each* group
```

N = 32 per group. The total N = 64.