

Estimators, tests and confidence intervals

STATISTIC AND ESTIMATOR

Recall: To learn something about the true unknown $\theta \in \Theta$ (or $\psi(\theta)$) we can only use the data $X \sim f_\theta$.

Definition 1.2

Let \mathcal{S} be any set. A (measurable) **function** $S : \mathcal{X} \rightarrow \mathcal{S}$ is called a **statistic**. If $\mathcal{S} = \Theta$ (or $\mathcal{S} = \Psi$) then S is called an **estimator** of θ (or of $\psi(\theta)$).

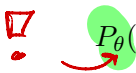
NOTATION

Let $\mathcal{M} = (\mathcal{X}, \Theta, \{f_\theta : \theta \in \Theta\})$ be a model.


$$\mathbb{E}(X) = ?$$

$$\Theta = ?$$

For an event $A \subseteq \mathcal{X}$ we write


$$P_\theta(A) := \begin{cases} \int_A f_\theta(x) dx & \text{if } f_\theta \text{ is a pdf,} \\ \sum_{x \in A} f_\theta(x) & \text{if } f_\theta \text{ is a pmf.} \end{cases}$$

For a statistic $S : \mathcal{X} \rightarrow \mathbb{R}$ we write


$$\mathbb{E}_\theta[S] := \begin{cases} \int_{\mathcal{X}} S(x) f_\theta(x) dx & \text{if } f_\theta \text{ is a pdf,} \\ \sum_{x \in \mathcal{X}} S(x) f_\theta(x) & \text{if } f_\theta \text{ is a pmf.} \end{cases}$$

Note: $X \sim f_\theta \Rightarrow \mathbb{E}[S(X)] = \mathbb{E}_\theta[S]$.

Analogously for $\text{Var}_\theta[S] = \text{Var}[S(X)]$,
 $\text{Cov}_\theta(S_1, S_2) = \text{Cov}(S_1(X), S_2(X))$.

EXAMPLE

$$X_1, \dots, X_n \stackrel{iid}{\sim} N(\mu, \sigma^2), \quad \theta = (\mu, \sigma^2) \in \mathbb{R} \times (0, \infty) =: \Theta.$$

that is, $f_\theta(x) = (2\pi\sigma^2)^{-n/2} \exp\left(-\frac{1}{2} \sum_{i=1}^n \frac{(x_i - \mu)^2}{\sigma^2}\right)$, $\mathcal{X} = \mathbb{R}^n$.

$$\hat{\mu}_n(x) := \frac{1}{n} \sum_{i=1}^n x_i, \quad x = (x_1, \dots, x_n)' \in \mathbb{R}^n$$

$$\hat{\sigma}_n^2(x) := \frac{1}{n-1} \sum_{i=1}^n (x_i - \hat{\mu}_n(x))^2$$

(Handwritten note: $\hat{\mu}_n(x)$ with an arrow pointing to the term in the sum)

$$\mathbb{E}_\theta[\hat{\mu}_n] = \mathbb{E}[\hat{\mu}_n(X_1, \dots, X_n)] = \frac{1}{n} \sum_{i=1}^n \mathbb{E}[X_i] = \mu, \quad \forall \theta \in \Theta.$$

" $\hat{\mu}_n$ is an unbiased estimator of μ "

$$\mathbb{E}_\theta[\hat{\sigma}_n^2] = \dots = \sigma^2, \quad \forall \theta \in \Theta. \quad \text{HW}$$

" $\hat{\sigma}_n^2$ is an unbiased estimator of σ^2 "

$$\text{Cov}_\theta(\hat{\mu}_n, \hat{\sigma}_n^2) = \dots = 0, \quad \forall \theta \in \Theta$$

HW

MAXIMUM LIKELIHOOD ESTIMATION

Consider a statistical model

$$\mathcal{M} = (\mathcal{X}, \Theta, \{f_\theta : \theta \in \Theta\})$$

Then $\theta \mapsto L(\theta|x) := f_\theta(x)$ is called the **likelihood function** and

$$\hat{\theta}(x) := \operatorname{argmax}_{\theta \in \Theta} L(\theta|x), \quad x \in \mathcal{X},$$

is called the **maximum likelihood estimator**.

If \mathcal{M} is an **iid model** with sample size n , we often maximize the **log-likelihood**

$$\Theta \mapsto \ell_n(\theta|x) := \log L(\theta|x) = \log \prod_{i=1}^n p_\theta(x_i) = \sum_{i=1}^n \log p_\theta(x_i).$$

This is equivalent, because

$$L(\theta|x) \leq L(\hat{\theta}(x)|x) \quad \forall \theta \in \Theta \iff \log L(\theta|x) \leq \log L(\hat{\theta}(x)|x) \quad \forall \theta \in \Theta.$$

EXAMPLE: ERDÖS-RÉNYI MODEL

$$N = \frac{n(n-1)}{2} \quad f_{\theta}(A) = \prod_{\substack{i,j=1 \\ i < j}}^n \theta^{A_{ij}} (1-\theta)^{1-A_{ij}} = \theta^{N \cdot \bar{A}_n} \cdot (1-\theta)^{N - N \cdot \bar{A}_n}$$

$$\hat{\theta} = \bar{A}_n = \frac{1}{N} \sum_{\substack{i,j=1 \\ i < j}}^n A_{ij}$$

$$L(\theta | A) = f_{\theta}(A)$$

$$\ell_N(\theta | A) = \log L(\theta | A) = N \cdot \bar{A}_n \log \theta +$$

$$+ N(1 - \bar{A}_n) \log(1 - \theta)$$
$$\frac{\partial}{\partial \theta} \ell_N(\theta | A) = \frac{N \cdot \bar{A}_n}{\theta} - \frac{N(1 - \bar{A}_n)}{1 - \theta} \stackrel{?}{=} 0$$

$$\Rightarrow \hat{\theta} = \bar{A}_n$$

$$\frac{\partial^2}{\partial \theta^2} \ell_N(\theta | A) \stackrel{?}{\leq} 0$$

\Rightarrow concave

HYPOTHESIS TESTS

null hypotheses
alternative
↓ ↓
h₀ h₁

Let $\mathcal{M} = (\mathcal{X}, \Theta, \{f_\theta : \theta \in \Theta\})$ be a model and $\Theta = \Theta_0 \cup \Theta_1$
where $\Theta_0 \cap \Theta_1 = \emptyset$.

Based on data $X \sim f_\theta$, we want to decide whether $H_0 : \theta \in \Theta_0$
or $H_1 : \theta \in \Theta_1$.

\Rightarrow test function $\varphi : \mathcal{X} \rightarrow \{0, 1\}$

Can make a mistake!

probability of **type one error**: $P_\theta(\varphi = 1), \theta \in \Theta_0$

probability of **type two error**: $P_\theta(\varphi = 0), \theta \in \Theta_1$

SIGNIFICANCE TESTS

Let $\mathcal{M} = (\mathcal{X}, \Theta, \{f_\theta : \theta \in \Theta\})$ be a model and $\Theta = \Theta_0 \cup \Theta_1$ where $\Theta_0 \cap \Theta_1 = \emptyset$.

Common approach: Fix a **significance level** $\alpha \in (0, 1)$ to control the probability of a type one error.

Definition 1.3

$\varphi_\alpha : \mathcal{X} \rightarrow \{0, 1\}$ is a **level α** test if

$$P_\theta(\varphi_\alpha = 1) \leq \alpha, \quad \forall \theta \in \Theta_0.$$

trivial level α test:

$$\varphi_0(x) = 0, \forall x \in \mathcal{X} \quad \Rightarrow \quad P_\theta(\varphi_0 = 1) = 0 \leq \alpha, \forall \theta \in \Theta_0.$$

Power:

$$\theta \in \Theta_1 \quad P_\theta(\varphi_\alpha = 1) = P_\theta(\emptyset) = 0$$

CRITICAL VALUE

Most of the time, a level α test will be of the form

$$\varphi_{\alpha}(x) = \begin{cases} 1, & \text{if } S(x) \geq c_{\alpha}, \\ 0, & \text{else,} \end{cases} \quad (1)$$

for some **test statistic** $S : \mathcal{X} \rightarrow \mathbb{R}$ and some **critical value** $c_{\alpha} \in \mathbb{R}$. (practical + theoretical reasons)

Ideally, we would like to take c_{α} such that

$$\sup_{\theta \in \Theta_0} P_{\theta}(\varphi_{\alpha} = 1) = \sup_{\theta \in \Theta_0} P_{\theta}(S \geq c_{\alpha}) \stackrel{!}{=} \alpha.$$

$\leq \alpha$

$$c < c_{\alpha} : P_{\theta}(S \geq c) > P_{\theta}(S \geq c_{\alpha}) = \alpha$$

$$c > c_{\alpha} : P_{\theta}(S \geq c) < P_{\theta}(S \geq c_{\alpha}) = \alpha \quad \theta \in \Theta_0$$

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ABSTRACT

BACKGROUND

Although several therapeutic agents have been evaluated for the treatment of coronavirus disease 2019 (Covid-19), no antiviral agents have yet been shown to be efficacious.

METHODS

We conducted a double-blind, randomized, placebo-controlled trial of intravenous remdesivir in adults who were hospitalized with Covid-19 and had evidence of lower respiratory tract infection. Patients were randomly assigned to receive either remdesivir (200 mg loading dose on day 1, followed by 100 mg daily for up to 9 additional days) or placebo for up to 10 days. The primary outcome was the time to recovery, defined by either discharge from the hospital or hospitalization for infection-control purposes only.

RESULTS

A total of 1062 patients underwent randomization (with 541 assigned to remdesivir and 521 to placebo). Those who received remdesivir had a median recovery time of 10 days (95% confidence interval [CI], 9 to 11), as compared with 15 days (95% CI, 13 to 18) among those who received placebo (rate ratio for recovery, 1.29; 95% CI, 1.12 to 1.49; $P < 0.001$, by a log-rank test). In an analysis that used a proportional-odds model with an eight-category ordinal scale, the patients who received remdesivir were found to be more likely than those who received placebo to have clinical improvement at day 15 (odds ratio, 1.5; 95% CI, 1.2 to 1.9, after adjustment for actual disease severity). The Kaplan–Meier estimates of mortality were 6.7% with remdesivir and 11.9% with placebo by day 15 and 11.4% with remdesivir and 15.2% with placebo by day 29 (hazard ratio, 0.73; 95% CI, 0.52 to 1.03). Serious

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EXAMPLE: THE TWO SAMPLE z -TEST

Consider the (simple) model:

- ▶ Treatment group: $X_1, \dots, X_{n_1} \stackrel{iid}{\sim} N(\mu_{treat}, 1), \mu_{treat} \geq 0$
- ▶ Control group: $Y_1, \dots, Y_{n_2} \stackrel{iid}{\sim} N(\mu_{cont}, 1), \mu_{cont} \geq 0$
- ▶ Treatment and control groups are independent
- ▶ Equal and known variances (for simplicity!). 🦄

X_i, Y_j are observed times to recovery.

$$H_0 : \mu_{cont} \leq \mu_{treat} \quad \text{vs.} \quad H_1 : \mu_{cont} > \mu_{treat}$$

$$\mathcal{X}, \Theta, f_\theta, \Theta_0, \Theta_1 ?$$

EXAMPLE: THE TWO SAMPLE z -TEST

- ▶ Treatment group: $X_1, \dots, X_{n_1} \stackrel{iid}{\sim} N(\mu_t, 1), \mu_t \geq 0$
- ▶ Control group: $Y_1, \dots, Y_{n_2} \stackrel{iid}{\sim} N(\mu_c, 1), \mu_c \geq 0$

$$H_0 : \mu_c \leq \mu_t \quad \text{vs.} \quad H_1 : \mu_c > \mu_t$$

Test statistic:

$$S = \frac{\bar{Y}_{n_2} - \bar{X}_{n_1}}{\sqrt{\frac{1}{n_1} + \frac{1}{n_2}}}.$$

S follows a normal distribution with mean

$$\Delta_\mu := \frac{\mu_c - \mu_t}{\sqrt{\frac{1}{n_1} + \frac{1}{n_2}}}$$

and unit variance:

$$S \sim N(\Delta_\mu, 1).$$

EXAMPLE: THE TWO SAMPLE z -TEST

$$H_0 : \mu_c \leq \mu_t \quad \text{vs.} \quad H_1 : \mu_c > \mu_t$$

or

$$H_0 : \Delta_\mu \leq 0 \quad \text{vs.} \quad H_1 : \Delta_\mu > 0$$

$$S \sim N(\Delta_\mu, 1).$$

Test φ_α : Reject H_0 if $S \geq q_{1-\alpha}^{(N)}$ $1 - \alpha$ quantile

Type-I error probability: $H_0 : \theta \in \Theta_0 \quad (\iff \Delta_\mu \leq 0)$

$$\sup_{\theta \in \Theta_0} P_\theta(\varphi_\alpha = 1) = \alpha?$$

EXAMPLE: THE TWO SAMPLE z -TEST

$$H_0 : \mu_c \leq \mu_t \quad \text{vs.} \quad H_1 : \mu_c > \mu_t$$

or

$$H_0 : \Delta_\mu \leq 0 \quad \text{vs.} \quad H_1 : \Delta_\mu > 0$$

$$S \sim N(\Delta_\mu, 1).$$

Test φ_α : Reject H_0 if $S \geq q_{1-\alpha}^{(N)}$ $1 - \alpha$ quantile

Power: $\theta \in \Theta_1 \iff \Delta_\mu > 0$ $< \infty$

$$\begin{aligned} P_\theta(S \geq q_{1-\alpha}^{(N)}) &= P_\theta(S - \Delta_\mu \geq \overbrace{q_{1-\alpha}^{(N)} - \Delta_\mu}^{< 0}) \\ &> P_\theta(S - \Delta_\mu \geq q_{1-\alpha}^{(N)}) = \alpha. \end{aligned}$$

p -VALUE

The p -value is the smallest significance level at which the test still rejects H_0 .

Definition 1.4

If, for every $\alpha \in (0, 1)$, φ_α is a level α test for $H_0 : \theta \in \Theta_0$, then the associated **p -value** is defined to be

$$p(x) := \inf\{\alpha \in (0, 1) : \varphi_\alpha(x) = 1\}, \quad x \in \mathcal{X}.$$

p -VALUE

Theorem 1.5

If, for every $\alpha \in (0, 1)$, φ_α is a level α test of $H_0 : \theta \in \Theta_0$ and $\varphi_\alpha(x) \leq \varphi_{\alpha'}(x)$ for all $x \in \mathcal{X}$ and all $\alpha \leq \alpha'$, then

$$\sup_{\theta \in \Theta_0} P_\theta(p \leq u) \leq u, \quad \forall u \in (0, 1).$$

Thus, the p -value can be used to perform a significance test at arbitrary level $u \in (0, 1)$:

$$\tilde{\varphi}_u(x) := \begin{cases} 1, & \text{if } p(x) \leq u, \\ 0, & \text{else.} \end{cases}$$

p -VALUE

Theorem 1.6

If the level α test φ_α of $H_0 : \theta \in \Theta_0$ is of the form (1) with $\sup_{\theta \in \Theta_0} P_\theta(S \geq c_\alpha) = \alpha$ for all $\alpha \in (0, 1)$ and $\alpha \mapsto c_\alpha$ is continuous and strictly decreasing, then

$$p(x) = \sup_{\theta \in \Theta_0} P_\theta(S \geq S(x)), \quad x \in \mathcal{X}.$$

In particular, if $\Theta_0 = \{\theta_0\}$, then

$$p(x) = P_{\theta_0}(S \geq S(x)), \quad x \in \mathcal{X}.$$

Thus, the p -value is the probability (under the null hypothesis) to observe the same or a more extreme value of the test statistic S than we actually did.

Note: The p -value quantifies the evidence against the null hypothesis.

EXAMPLE: THE TWO SAMPLE z -TEST

$$H_0 : \mu_c \leq \mu_t \quad \text{vs.} \quad H_1 : \mu_c > \mu_t$$

or

$$H_0 : \Delta_\mu \leq 0 \quad \text{vs.} \quad H_1 : \Delta_\mu > 0$$

$$S \sim N(\Delta_\mu, 1).$$

Test φ_α : Reject H_0 if $S \geq q_{1-\alpha}^{(N)}$ $1 - \alpha$ quantile

p-value: (cf. Theorem 1.6)

$$\sup_{\theta \in \Theta_0} P_\theta(S \geq s)$$

$$p = \sup_{\theta \in \Theta_0} P_\theta(S \geq s) \Big|_{s=S} = \sup_{\Delta_\mu \leq 0} P(N(0, 1) \geq s - \Delta_\mu) \Big|_{s=S}$$

$$= P(N(0, 1) \geq s) \Big|_{s=S} = 1 - \Phi(S) \qquad \rho(x) = 1 - \Phi(S(x))$$

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CONFIDENCE INTERVALS (CI)

Definition 1.7

Let $\mathcal{M} = (\mathcal{X}, \Theta, \{f_\theta : \theta \in \Theta\})$ be a model and $\alpha \in (0, 1)$ be an **error probability**. A data dependent set $CS_\alpha(x) \subseteq \Theta$, $x \in \mathcal{X}$, is called a **level $1 - \alpha$ confidence set** for θ if

$$P_\theta(\theta \in CS_\alpha) \geq 1 - \alpha, \quad \forall \theta \in \Theta.$$

If $\Theta \subseteq \mathbb{R}$ and $CS_\alpha(x) = [l_\alpha(x), u_\alpha(x)]$ is an interval, then $CI_\alpha := CS_\alpha$ is called a **confidence interval** for θ .

trivial level $1 - \alpha$ CI: ($\Theta \subseteq \mathbb{R}$)

$$CI_0(x) := \mathbb{R} \quad \Rightarrow \quad P_\theta(\theta \in CI_0) = P_\theta(\mathcal{X}) = 1 \geq 1 - \alpha, \quad \forall \theta \in \Theta.$$

Length of the CI: $\text{length}(CI_\alpha(x)) = \infty$

DUALITY OF TESTS AND CONFIDENCE SETS

Theorem 1.8

Let $\mathcal{M} = (\mathcal{X}, \Theta, \{f_\theta : \theta \in \Theta\})$ be a model and $\alpha \in (0, 1)$ be an **error probability**.

- a) If, for every $\theta_0 \in \Theta$, $\varphi_{\alpha, \theta_0} : \mathcal{X} \rightarrow \{0, 1\}$ is a level α test for $H_0 : \theta = \theta_0$ (i.e., $\Theta_0 = \{\theta_0\}$), then

$$CS_\alpha(x) := \{\theta \in \Theta : \varphi_{\alpha, \theta}(x) = 0\}$$

is a level $1 - \alpha$ confidence set for θ .

- b) If $CS_\alpha(x) \subseteq \Theta$ is a level $1 - \alpha$ confidence set for θ , then

$$\varphi_\alpha(x) := \begin{cases} 1, & \text{if } \Theta_0 \cap CS_\alpha(x) = \emptyset, \\ 0, & \text{else,} \end{cases}$$

is a level α test for $H_0 : \theta \in \Theta_0$.

THE TWO SAMPLE z -TEST

- ▶ Treatment group: $X_1, \dots, X_{n_1} \stackrel{iid}{\sim} N(\mu_t, 1), \mu_t \geq 0$
- ▶ Control group: $Y_1, \dots, Y_{n_2} \stackrel{iid}{\sim} N(\mu_c, 1), \mu_c \geq 0$

$$H_0 : \mu_c \leq \mu_t \quad \text{vs.} \quad H_1 : \mu_c > \mu_t$$

Test statistic:

$$S = \frac{\bar{Y}_{n_2} - \bar{X}_{n_1}}{\sqrt{\frac{1}{n_1} + \frac{1}{n_2}}} \sim N(\Delta_\mu, 1), \quad \Delta_\mu := \frac{\mu_c - \mu_t}{\sqrt{\frac{1}{n_1} + \frac{1}{n_2}}} \in \mathbb{R}.$$

$$H_0 : \Delta_\mu \leq 0 \quad \text{vs.} \quad H_1 : \Delta_\mu > 0$$

Δ_μ ... effect size

Want a confidence interval for Δ_μ .

CI FOR THE MEAN OF A NORMAL DISTRIBUTION

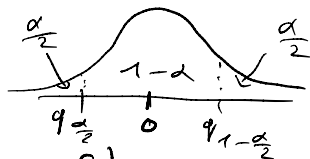
Model

$$S \sim N(\Delta, 1), \quad \Delta \in \mathbb{R}.$$

Want a confidence interval for the effect size Δ .

$$S - \Delta \sim N(0, 1)$$

$$1 - \alpha = P_{\Delta} \left(q_{\alpha/2}^{(N)} \leq S - \Delta \leq q_{1-\alpha/2}^{(N)} \right)$$



$$= P_{\Delta} \left(q_{\alpha/2} - S \leq -\Delta \leq q_{1-\alpha/2} - S \right)$$

$$= P_{\Delta} \left(S - \underbrace{q_{\alpha/2}}_{= -q_{1-\alpha/2}} \geq \Delta \geq S - q_{1-\alpha/2} \right)$$

$$= P_{\Delta} \left(\Delta \in CI_{\alpha} \right)$$

$$CI_{\alpha} = [S - q_{1-\alpha/2}, S + q_{1-\alpha/2}]$$

CI FOR THE MEAN OF A NORMAL DISTRIBUTION

$$S \sim N(\Delta, 1), \quad \Delta \in \mathbb{R}.$$

$$CI_\alpha(S) = [S - q_{1-\alpha/2}^{(N)}, S + q_{1-\alpha/2}^{(N)}]$$

Turn this into a test for

$$H_0 : \Delta \leq 0 \quad \text{vs.} \quad H_1 : \Delta > 0,$$

using Theorem 1.8:

$$\varphi_\alpha(S) = \begin{cases} 1, & \text{if } \Theta_0 \cap CI_\alpha(S) = \emptyset \\ 0, & \text{else,} \end{cases}$$

CI FOR THE MEAN OF A NORMAL DISTRIBUTION

$$S \sim N(\Delta, 1), \quad \Delta \in \mathbb{R}.$$

$$CI_\alpha(S) = [S - q_{1-\alpha}^{(N)}, \infty)$$

Turn this into a test for

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