



Statistical Modelling

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Welcome

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This course is about statistical modelling.

A famous quote attributed to George Box claims that

All models are wrong, but some are useful.

This standpoint is reductive: Peter McCullagh and John Nelder wrote in the preamble of their book (emphasis mine)

Modelling in science remains, partly at least, an art. Some principles do exist, however, to guide the modeller. The first is that all models are wrong; **some, though, are better** than others and we can **search for the better ones**. At the same time we must recognize that eternal truth is not within our grasp.

And this quote by David R. Cox adds to the point:

... it does not seem helpful just to say that all models are wrong. The very word model implies simplification and idealization. The idea that complex physical, biological or sociological systems can be exactly described by a few formulae is patently absurd. The construction of idealized representations that **capture important stable aspects of such systems** is, however, a vital part of general scientific analysis and statistical models, especially substantive ones, do not seem essentially different from other kinds of model.

Why use models? Paul Krugman wrote in 2010 in his blog

The answer I'd give is that models are an enormously important tool for clarifying your thought. You don't have to literally believe your model — in fact, you're a fool if you do — to believe that putting together a simplified but complete account of how things work, with all the eyes crossed and teas dotted or something, helps you gain a much more sophisticated understanding of the real situation. People who don't use models end up relying on slogans that are much more simplistic than the models

Course content

There are two main data type: **experimental** data are typically collected in a control environment following a research protocol with a particular experimental design: they serve to answer questions specified ahead of time. This approach is highly desirable to avoid the garden of forking paths (researchers unfortunately tend to refine or change their hypothesis in light of data, which invalidates their findings — preregistration alleviates this somewhat). While experimental data are highly desirable, it is not always possible to collect experimental data: for example, an economist cannot modify interest rates to see how it impacts consumer savings. When data have been collected beforehand without intervention (for other purposes), these are called **observational**. These will be the ones most frequently encountered.

A stochastic model will comprise two ingredients: a distribution for the random data and a formula linking the parameters or the conditional expectation of a response variable Y to a set of explanatories X . A model can serve to either predict new outcomes (predictive modelling) or else to test research hypothesis about the effect of the explanatory variables on the response (explanatory model). These two objectives are of course not mutually exclusive even if we distinguish in practice inference and prediction.

A predictive model gives predictions of Y for different combinations of explanatory variables or future data. For example, one could try to forecast the energy consumption of a house as a function of weather, the number of inhabitants and its size. Black boxes used in machine learning are often used solely for prediction: these models are not easily interpreted and they often ignore the data structure.

By contrast, explicative models are often simple and interpretable: regression models are often used for inference purpose and we will focus on these. The following examples will be covered in class or as part of the exercises:

- Are sequential decisions in online shop (buying or not, then selecting the quantity) preferable to integrated decisions (Duke and Amir 2023)?
- Determining what is the most distracting for road users: talking on a cellphone, texting or checking your smartwatch (Brodeur et al. 2021)?
- What is the impact of inconsistencies between product description and the displayed image (Lee and Choi 2019)?
- Is the price of gasoline more expensive in the Gaspé peninsula than in the rest of Quebec? A report of the *Régie de l'énergie* examines the question
- Are driving tests in the UK easier if you live in a rural area? An analysis of *The Guardian* hints that it is the case.
- What is the environmental perception of a package that includes cardboard over a plastic container (Sokolova, Krishna, and Döring 2023)?

- What is the psychological impact of suggested amounts on donations (Moon and VanEpps 2023)?
- What are the benefits of face-to-face meetings, rather than via videoconference tools? Brucks and Levav (2022) suggests a decrease in the number of creative ideas and interactions when meeting online.

1 Introduction

This chapter reviews some basic notions of probability and statistics that are normally covered in undergraduate or college.

1.1 Population and samples

Statistics is the science of uncertainty quantification: of paramount importance is the notion of randomness. Generally, we will seek to estimate characteristics of a population using only a sample (a sub-group of the population of smaller size).

The **population of interest** is a collection of individuals which the study targets. For example, the Labour Force Survey (LFS) is a monthly study conducted by Statistics Canada, who define the target population as “all members of the selected household who are 15 years old and older, whether they work or not.” Asking every Canadian meeting this definition would be costly and the process would be long: the characteristic of interest (employment) is also a snapshot in time and can vary when the person leaves a job, enters the job market or become unemployed.

In general, we therefore consider only **samples** to gather the information we seek to obtain. The purpose of **statistical inference** is to draw conclusions about the population, but using only a share of the latter and accounting for sources of variability. George Gallup made this great analogy between sample and population:

One spoonful can reflect the taste of the whole pot, if the soup is well-stirred

A **sample** is a random sub-group of individuals drawn from the population. Creation of sampling plans is a complex subject and semester-long sampling courses would be required to even scratch the surface of the topic. Even if we won't be collecting data, keep in mind the following information: for a sample to be good, it must be representative of the population under study. Selection bias must be avoided, notably samples of friends or of people sharing opinions.

Because the individuals are selected at **random** to be part of the sample, the measurement of the characteristic of interest will also be random and change from one sample

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to the next. However, larger samples of the same quality carry more information and our estimator will be more precise. Sample size is not guarantee of quality, as the following example demonstrates.

Example 1.1 (Polling for the 1936 USA Presidential Election). *The Literary Digest* surveyed 10 millions people by mail to know voting preferences for the 1936 USA Presidential Election. A sizeable share, 2.4 millions answered, giving Alf Landon (57%) over incumbent President Franklin D. Roosevelt (43%). The latter nevertheless won in a landslide election with 62% of votes cast, a 19% forecast error. Biased sampling and differential non-response are mostly responsible for the error: the sampling frame was built using “phone number directories, drivers’ registrations, club memberships, etc.”, all of which skewed the sample towards rich upper class white people more susceptible to vote for the GOP.

In contrast, Gallup correctly predicted the outcome by polling (only) 50K inhabitants. Read the full story [here](#).

1.1.1 Variable type

- a **variable** represents a characteristic of the population, for example the sex of an individual, the price of an item, etc.
- an **observation** is a set of measures (variables) collected under identical conditions for an individual or at a given time.

Table 1.1: First lines of the `renfe` database, which contains the price of 10K train tickets between Madrid and Barcelona. The columns `price` and `duration` represent continuous variables, all others are categorical.

price	type	class	fare	dest	duration	wday
143.4	AVE	Preferente	Promo	Barcelona-Madrid	190	6
181.5	AVE	Preferente	Flexible	Barcelona-Madrid	190	2
86.8	AVE	Preferente	Promo	Barcelona-Madrid	165	7
86.8	AVE	Preferente	Promo	Barcelona-Madrid	190	7
69.0	AVE-TGV	Preferente	Promo	Barcelona-Madrid	175	4

The choice of statistical model and test depends on the underlying type of the data collected. There are many choices: quantitative (discrete or continuous) if the variables are numeric, or qualitative (binary, nominal, ordinal) if they can be described using an adjective; I prefer the term categorical, which is more evocative.

Most of the models we will deal with are so-called regression models, in which the mean of a quantitative variable is a function of other variables, termed explanatories. There are two types of numerical variables

- a discrete variable takes a finite or countable number of values, prime examples being binary variables or count variables.
- a continuous variable can take (in theory) an infinite possible number of values, even when measurements are rounded or measured with a limited precision (time, width, mass). In many case, we could also consider discrete variables as continuous if they take enough values (e.g., money).

Categorical variables take only a finite of values. They are regrouped in two groups,

- nominal if there is no ordering between levels (sex, color, country of origin) or
- ordinal if they are ordered (Likert scale, salary scale) and this ordering should be reflected in graphs or tables.

We will bundle every categorical variable using arbitrary encoding for the levels: for modelling, these variables taking K possible values (or levels) must be transformed into a set of $K - 1$ binary 0/1 variables, the omitted level corresponding to a baseline. Failing to declare categorical variables in your favorite software is a common mistake, especially when these are saved in the database using integers rather than strings.

1.2 Random variable

Suppose we wish to describe the behaviour of a stochastic phenomenon. To this effect, one should enumerate the set of possible values taken by the variable of interest and their probability: this is what is encoded in the distribution.

Random variables are denoted using capital letters: for example $Y \sim \text{normal}(\mu, \sigma^2)$ indicates that Y follows a normal distribution with parameters μ and $\sigma > 0$. If the values of the latter are left unspecified, we talk about the family of distributions. When the values are given, for example $\mu = 0$ and $\sigma = 1$, we deal with a single distribution for which a function encode the probability of the underlying variable.

Definition 1.1 (Distribution function, mass function and density). The (cumulative) distribution function $F(y)$ gives the cumulative probability that an event doesn't exceed a given numerical value y , $F(y) = \Pr(Y \leq y)$.

If Y is discrete, then it has atoms of non-zero probability and we call f the mass function, and $f(y) = \Pr(Y = y)$ gives the probability of each outcome y . In the continuous case,

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no numerical value has non-zero probability and so we consider intervals instead. The density function $f(x)$ is non-negative and satisfies $\int_{\mathbb{R}} f(x)dx = 1$: the integral over a set B (the area under the curve) gives the probability of Y falling inside $B \in \mathbb{R}$. It follows that the distribution function of a continuous random variable is simply $F(y) = \int_{-\infty}^y f(x)dx$.

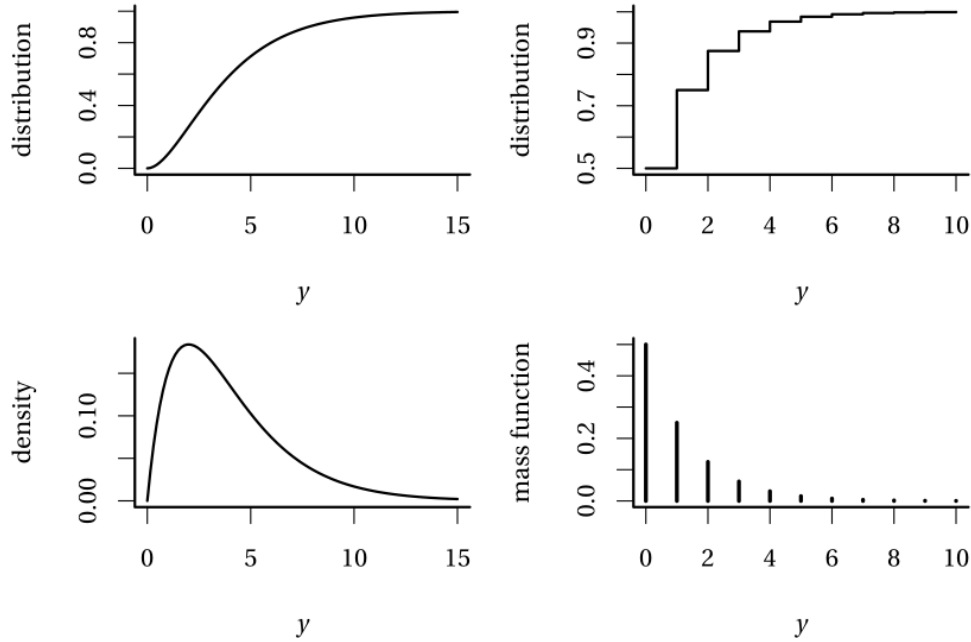


Figure 1.1: (Cumulative) distribution functions (top) and density/mass functions (bottom) of continuous (left) and discrete (right) random variables.

One of the first topics covered in introductory statistics is descriptive statistics such as the mean and standard deviation. These are estimators of (centered) moments, which characterise a random variable. In the case of the standard normal distribution, the expectation and variance fully characterize the distribution.

Definition 1.2 (Moments). Let Y be a random variable with density (or mass) function $f(x)$. The **expectation** (or theoretical mean) of a continuous random variable Y is

$$E(Y) = \int_{\mathbb{R}} xf(x)dx.$$

In the discrete case, we set rather $\mu = E(Y) = \sum_{x \in \mathcal{X}} x\Pr(X = x)$, where \mathcal{X} denotes the support of Y , the set of numerical values at which the probability of Y is non-zero. More generally, we can look at the expectation of a function $g(x)$ for Y , which is nothing but the

integral (or sum in the discrete case) of $g(x)$ weighted by the density or mass function of $f(x)$. In the same fashion, provided the integral is finite, the variance is

$$\text{Va}(Y) = \mathbb{E}\{Y - \mathbb{E}(Y)\}^2 \equiv \int_{\mathbb{R}} (x - \mu)^2 f(x) dx.$$

The **standard deviation** is the square root of the variance, $\text{sd}(Y) = \sqrt{\text{Va}(Y)}$: its units are the same as those of Y and are thus more easily interpreted.

The notion of moments can be extended to higher dimensions. Consider an n -vector \mathbf{Y} . In the regression setting, the response \mathbf{Y} would usually comprise repeated measures on an individual, or even observations from a group of individuals.

The expected value (theoretical mean) of the vector \mathbf{Y} is calculated componentwise, i.e.,

$$\mathbb{E}(\mathbf{Y}) = \boldsymbol{\mu} = \left(\mathbb{E}(Y_1) \quad \cdots \quad \mathbb{E}(Y_n) \right)^\top$$

whereas the second moment of \mathbf{Y} is encoded in the $n \times n$ **covariance** matrix

$$\text{Va}(\mathbf{Y}) = \boldsymbol{\Sigma} = \begin{pmatrix} \text{Va}(Y_1) & \text{Co}(Y_1, Y_2) & \cdots & \text{Co}(Y_1, Y_n) \\ \text{Co}(Y_2, Y_1) & \text{Va}(Y_2) & \ddots & \vdots \\ \vdots & \ddots & \ddots & \vdots \\ \text{Co}(Y_n, Y_1) & \text{Co}(Y_n, Y_2) & \cdots & \text{Va}(Y_n) \end{pmatrix}$$

The i th diagonal element of $\boldsymbol{\Sigma}$, $\sigma_{ii} = \sigma_i^2$, is the variance of Y_i , whereas the off-diagonal entries $\sigma_{ij} = \sigma_{ji}$ ($i \neq j$) are the covariance of pairwise entries, with

$$\text{Co}(Y_i, Y_j) = \int_{\mathbb{R}^2} (y_i - \mu_i)(y_j - \mu_j) f_{Y_i, Y_j}(y_i, y_j) dy_i dy_j.$$

The covariance matrix $\boldsymbol{\Sigma}$ is thus symmetric. It is customary to normalize the pairwise dependence so they do not depend on the component variance. The linear **correlation** between Y_i and Y_j is

$$\rho_{ij} = \text{Cor}(Y_i, Y_j) = \frac{\text{Co}(Y_i, Y_j)}{\sqrt{\text{Va}(Y_i)}\sqrt{\text{Va}(Y_j)}} = \frac{\sigma_{ij}}{\sigma_i \sigma_j}.$$

The correlation matrix of \mathbf{Y} is an $n \times n$ symmetric matrix with ones on the diagonal and the pairwise correlations off the diagonal,

$$\text{Cor}(\mathbf{Y}) = \begin{pmatrix} 1 & \rho_{12} & \rho_{13} & \cdots & \rho_{1n} \\ \rho_{21} & 1 & \rho_{23} & \cdots & \rho_{2n} \\ \rho_{31} & \rho_{32} & 1 & \ddots & \rho_{3n} \\ \vdots & \vdots & \ddots & \ddots & \vdots \\ \rho_{n1} & \rho_{n2} & \rho_{n3} & \cdots & 1 \end{pmatrix}.$$

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One of the most important parts of modelling correlated (or longitudinal) data is the need to account for within-group correlations. This basically comes down to modelling a covariance matrix for observations within the same group (or within the same individual in the case of repeated measures), which is the object of Chapter 5.

Definition 1.3 (Bias). The bias of an estimator $\hat{\theta}$ for a parameter θ is

$$\text{bias}(\hat{\theta}) = E(\hat{\theta}) - \theta$$

The estimator is unbiased if its bias is zero.

Example 1.2 (Unbiased estimators). The unbiased estimator of the mean and the variance of Y are

$$\begin{aligned}\bar{Y}_n &= n^{-1} \sum_{i=1}^n Y_i \\ S_n &= (n-1)^{-1} \sum_{i=1}^n (Y_i - \bar{Y})^2.\end{aligned}$$

While unbiasedness is a desirable property, there may be cases where no unbiased estimator exists for a parameter! Often, rather, we seek to balance bias and variance: recall that an estimator is a function of random variables and thus it is itself random: even if it is unbiased, the numerical value obtained will vary from one sample to the next.

Definition 1.4. We often seek an estimator that minimises the **mean squared error**,

$$\text{MSE}(\hat{\theta}) = E\{(\hat{\theta} - \theta)^2\} = \text{Va}(\hat{\theta}) + \{E(\hat{\theta}) - \theta\}^2.$$

The mean squared error is an objective function consisting of the sum of the squared bias and the variance.

Most estimators we will consider are so-called maximum likelihood estimator. These estimator are asymptotically efficient, in the sense that they have the lowest mean squared error of all estimators for large samples. Other properties of maximum likelihood estimators also make them attractive default choice for estimation.

1.3 Discrete distributions

Many distributions for discrete random variables have a simple empirical justification, stemming from simple combinatorial arguments (counting). We revisit the most common ones.

Definition 1.5 (Bernoulli distribution). We consider a binary event such as coin toss (heads/tails). In general, the two events are associated with success/failure. By convention, failures are denoted by zeros and successes by ones, the probability of success being p so $\Pr(Y = 1) = p$ and $\Pr(Y = 0) = 1 - p$ (complementary event). The mass function of the Bernoulli distribution is thus

$$\Pr(Y = y) = p^y(1 - p)^{1-y}, \quad y = 0, 1.$$

A rapid calculation shows that $E(Y) = p$ and $Va(Y) = p(1 - p)$. Indeed,

$$E(Y) = E(Y^2) = p \cdot 1 + (1 - p) \cdot 0 = p.$$

Many research questions have binary responses, for example:

- did a potential client respond favourably to a promotional offer?
- is the client satisfied with service provided post-purchase?
- will a company go bankrupt in the next three years?
- did a study participant successfully complete a task?

Oftentimes, we will have access to aggregated data.

Definition 1.6 (Binomial distribution). If we consider the sum of independent and identically distributed Bernoulli events, the number of successes Y out of m trials is binomial, denoted $\text{Bin}(m, p)$; the mass function of the binomial distribution is

$$\Pr(Y = y) = \binom{m}{y} p^y (1 - p)^{m-y}, \quad y = 0, 1, \dots, m.$$

The likelihood of a sample from a binomial distribution is (up to a normalizing constant that doesn't depend on p) the same as that of m independent Bernoulli trials. The expectation of the binomial random variable is $E(Y) = mp$ and its variance $Va(Y) = mp(1 - p)$.

As examples, we could consider the number of successful candidates out of m who passed their driving license test or the number of customers out of m total which spent more than 10\$ in a store.

More generally, we can also consider count variables whose realizations are integer-valued, for examples the number of

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- insurance claims made by a policyholder over a year,
- purchases made by a client over a month on a website,
- tasks completed by a study participant in a given time frame.

Definition 1.7 (Poisson distribution). If the probability of success p of a Bernoulli event is small in the sense that $mp \rightarrow \lambda$ when the number of trials m increases, then the number of success follows approximately a Poisson distribution with mass function

$$\Pr(Y = y) = \frac{\exp(-\lambda)\lambda^y}{\Gamma(y+1)}, \quad y = 0, 1, 2, \dots$$

where $\Gamma(\cdot)$ denotes the gamma function. The parameter λ of the Poisson distribution is both the expectation and the variance of the distribution, meaning $E(Y) = \text{Va}(Y) = \lambda$.

Definition 1.8 (Negative binomial distribution). The negative binomial distribution arises if we consider the number of Bernoulli trials with probability of success p until we obtain m success. Let Y denote the number of failures: the order of success and failure doesn't matter, except for the latest trial which must be a success. The mass function of the negative binomial is

$$\Pr(Y = y) = \binom{m-1+y}{y} p^m (1-p)^y.$$

The negative binomial distribution also appears as the unconditional distribution of a two-stage hierarchical gamma-Poisson model, in which the mean of the Poisson distribution is random and follows a gamma distribution. In notation, this is $Y \mid \Lambda = \lambda \sim \text{Po}(\lambda)$ and Λ follows a gamma distribution with shape r and scale θ , whose density is

$$f(x) = \theta^{-r} x^{r-1} \exp(-x/\theta) / \Gamma(r).$$

The unconditional number of success is then negative binomial.

In the context of generalized linear models, we will employ yet another parametrisation of the distribution, with the mass function

$$\Pr(Y = y) = \frac{\Gamma(y+r)}{\Gamma(y+1)\Gamma(r)} \left(\frac{r}{r+\mu} \right)^r \left(\frac{\mu}{r+\mu} \right)^y, \quad y = 0, 1, \dots, \mu, r > 0,$$

where Γ is the gamma function and the parameter $r > 0$ is not anymore integer valued. The expectation and variance of Y are $E(Y) = \mu$ et $\text{Va}(Y) = \mu + k\mu^2$, where $k = 1/r$. The variance of the negative binomial distribution is thus higher than its expectation, which justifies the use of the negative binomial distribution for modelling overdispersion.

1.3.1 Continuous distributions

We will encounter many continuous distributions that arise as (asymptotic) null distribution of test statistics because of the central limit theorem, or that follow from transformation of Gaussian random variables.

Definition 1.9 (Beta distribution). The beta distribution $\text{Beta}(\alpha, \beta)$ is a distribution supported on the unit interval $[0, 1]$ with shape parameters $\alpha > 0$ and $\beta > 0$. Its density is

$$f(x) = \frac{\Gamma(\alpha)\Gamma(\beta)}{\Gamma(\alpha + \beta)} x^{\alpha-1} (1-x)^{1-\beta}, \quad x \in [0, 1].$$

The case $\alpha = \beta = 1$, also denoted $\text{unif}(0, 1)$, corresponds to a standard uniform distribution.

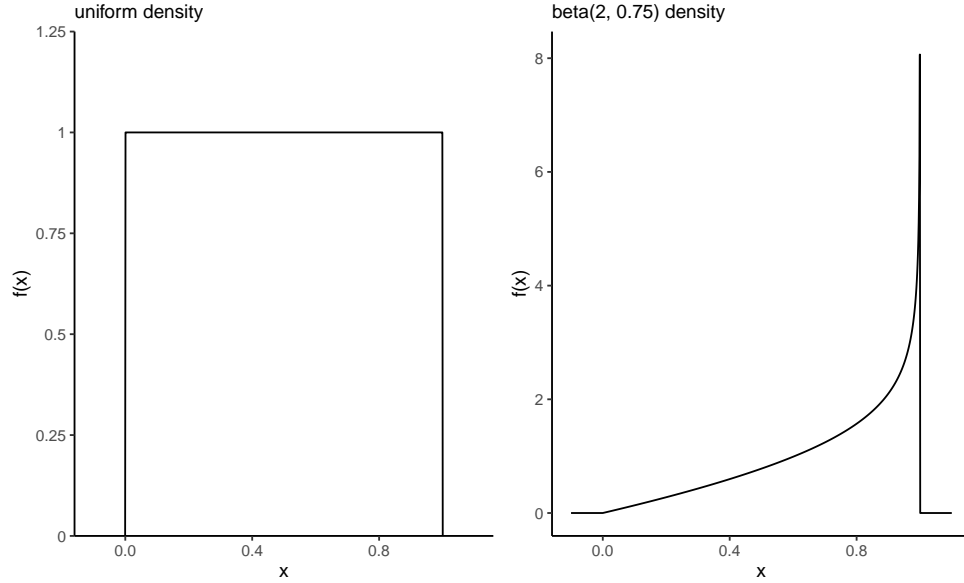


Figure 1.2: Density function of uniform (left) and beta(2, 3/4) random variables on the unit interval.

Definition 1.10 (Exponential distribution). The exponential distribution plays a prominent role in the study of waiting time of Poisson processes, and in survival analysis. One characteristic of the distribution is its absence of memory: $\Pr(Y \geq y + u \mid Y > u) = \Pr(Y > y)$ for $y, u > 0$.

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The distribution function of the exponential distribution, $Y \sim \text{Exp}(\beta)$, here parametrized in terms of scale $\beta > 0$ is $F(x) = 1 - \exp(-\beta x)$ and the corresponding density function is $f(x) = \beta \exp(-\beta x)$ for $x > 0$. The expected value of Y is simply β .

Definition 1.11 (Normal distribution). This most well known distribution, the normal distribution is ubiquitous in statistics because of the central limit theorem (CLT), which describes the behaviour of the sample mean in large sample. The parameters μ and $\sigma > 0$ that fully characterize the distribution of the normal distribution and they correspond to the expectation and standard deviation. The density of a normal distribution is symmetric around μ , while σ describes the dispersion around this mode. The bell-shaped density function is

$$f(x) = (2\pi\sigma^2)^{-1/2} \exp\left\{-\frac{(x - \mu)^2}{2\sigma^2}\right\}, \quad x \in \mathbb{R}.$$

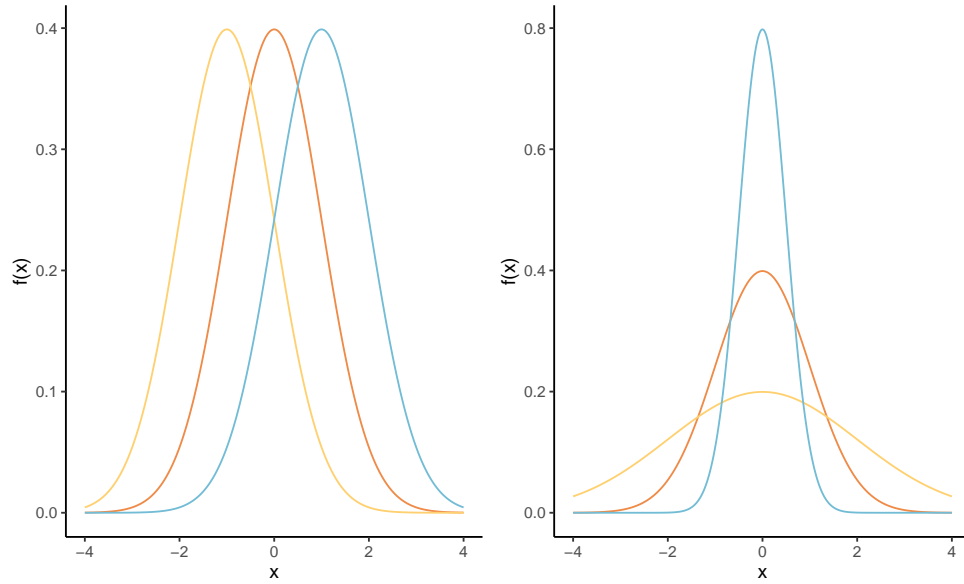


Figure 1.3: Densities of normal distributions with different mean parameters (left) and different scale parameters (right).

The distribution function of the normal distribution is not available in closed-form. The normal distribution is a location-scale distribution: if $Y \sim \text{normal}(\mu, \sigma^2)$, then $Z = (Y - \mu)/\sigma \sim \text{normal}(0, 1)$. Conversely, if $Z \sim \text{normal}(0, 1)$, then $Y = \mu + \sigma Z \sim \text{normal}(\mu, \sigma^2)$.

We will also encounter the multivariate normal distribution; for a d dimensional vector

$\mathbf{Y} \sim \text{normal}_d(\boldsymbol{\mu}, \boldsymbol{\Sigma})$, the density is

$$f(\mathbf{x}) = (2\pi)^{-d/2} |\boldsymbol{\Sigma}|^{-1/2} \exp \left\{ -\frac{1}{2} (\mathbf{x} - \boldsymbol{\mu})^\top \boldsymbol{\Sigma}^{-1} (\mathbf{x} - \boldsymbol{\mu}) \right\}$$

The mean vector $\boldsymbol{\mu}$ is the vector of expectation of individual observations, whereas $\boldsymbol{\Sigma}$ is the $d \times d$ covariance matrix of \mathbf{Y} . A unique property of the multivariate normal distribution is the link between independence and the covariance matrix: if Y_i and Y_j are independent, the (i, j) off-diagonal entry of $\boldsymbol{\Sigma}$ is zero.

Definition 1.12 (Chi-square distribution). The chi-square distribution with $\nu > 0$ degrees of freedom, denoted χ_ν^2 or chi – square(ν). It's density is

$$f(x; \nu) = \frac{1}{2^{\nu/2} \Gamma(\nu/2)} x^{\nu/2-1} \exp(-x/2), \quad x > 0.$$

It can be obtained for ν integer by considering the following: if we consider k independent and identically distributed standard normal variables, $Y_i \sim \text{normal}(0, 1)$, then $\sum_{i=1}^k Y_i^2$ follows a chi-square distribution with k degrees of freedom, denote χ_k^2 . The square of a standard normal variate likewise follows a χ_1^2 distribution. The expectation of χ_k^2 random variable is k .

If we consider a sample of n normally distributed observations, the scaled sample variance $(n-1)S^2/\sigma^2 \sim \chi_{n-1}^2$.

Definition 1.13 (Student- t distribution). The Student- t distribution with $\nu > 0$ degrees of freedom is a location-scale family. The standard version is denoted by Student(ν).

The name “Student” comes from the pseudonym used by William Gosset in Gosset (1908), who introduced the asymptotic distribution of the t -statistic. The density of the standard T with ν degrees of freedom is

$$f(y; \nu) = \frac{\Gamma\left(\frac{\nu+1}{2}\right)}{\Gamma\left(\frac{\nu}{2}\right) \sqrt{\nu\pi}} \left(1 + \frac{y^2}{\nu}\right)^{-\frac{\nu+1}{2}}$$

the distribution has polynomial tails, is symmetric around 0 and unimodal. As $\nu \rightarrow \infty$, the Student distribution converges to a normal distribution. It has heavier tails than the normal distribution and only the first $\nu - 1$ moments of the distribution exist, so a Student distribution with $\nu = 2$ degrees of freedom has infinite variance.

For normally distributed data, the centered sample mean divided by the sample variance, $(\bar{Y} - \mu)/S^2$ follows a Student- t distribution with $n - 1$ degrees of freedom, which explains the terminology t -tests.

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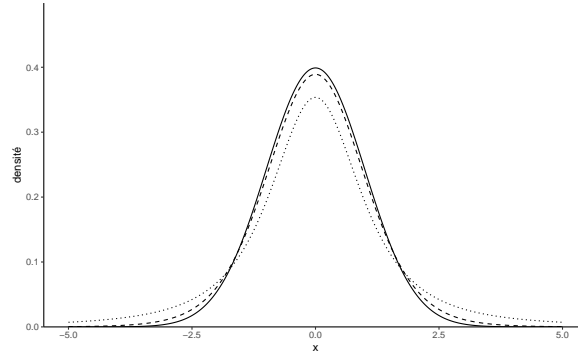


Figure 1.4: Comparison between the Student- t density for varying degrees of freedom, with $\nu = 2$ (dotted), $\nu = 10$ (dashed) and the normal density ($\nu = \infty$).

Definition 1.14 (Fisher distribution). The Fisher or F distribution is used to determine the large sample behaviour of test statistics for comparing different group averages (in analysis of variance) assuming data are normally distributed.

The F distribution, denoted $\text{Fisher}(\nu_1, \nu_2)$, is obtained by dividing two independent chi-square random variables with respective degrees of freedom ν_1 and ν_2 . Specifically, if $Y_1 \sim \chi_{\nu_1}^2$ and $Y_2 \sim \chi_{\nu_2}^2$, then

$$F = \frac{Y_1/\nu_1}{Y_2/\nu_2} \sim \text{Fisher}(\nu_1, \nu_2)$$

The Fisher distribution tends to a $\chi_{\nu_1}^2$ when $\nu_2 \rightarrow \infty$.

1.4 Graphs

This section reviews the main graphical representation of random variables, depending on their type.

The main type of graph for representing categorical variables is bar plot (and modifications thereof). In a bar plot, the frequency of each category is represented in the y -axis as a function of the (ordered) levels on the x -axis. This representation is superior to the ignominious pie chart, a nuisance that ought to be banned (humans are very bad at comparing areas and a simple rotation changes the perception of the graph)!

Continuous variables can take as many distinct values as there are observations, so we cannot simply count the number of occurrences by unique values. Instead, we bin them

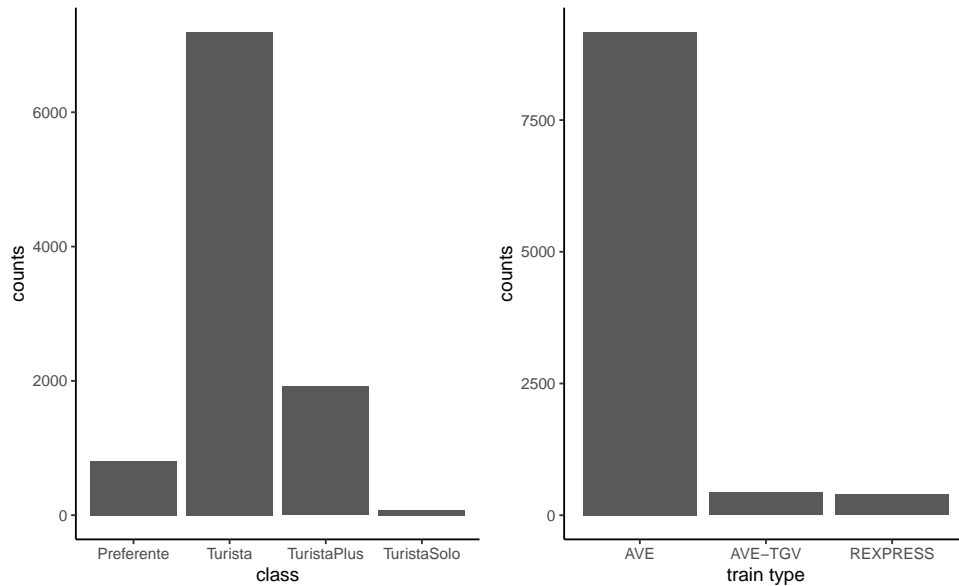


Figure 1.5: Bar plot of ticket class for Renfe tickets data

into distinct intervals so as to obtain an histogram. The number of class depends on the number of observations: as a rule of thumb, the number of bins should not exceed \sqrt{n} , where n is the sample size. We can then obtain the frequency in each class, or else normalize the histogram so that the area under the bands equals one: this yields a discrete approximation of the underlying density function. Varying the number of bins can help us detect patterns (rounding, asymmetry, multimodality).

Since we bin observations together, it is sometimes difficult to see where they fall. Adding rugs below or above the histogram will add observation about the range and values taken, where the heights of the bars in the histogram carry information about the (relative) frequency of the intervals.

If we have a lot of data, it sometimes help to focus only on selected summary statistics.

Definition 1.15 (Box-and-whiskers plot). A box-and-whiskers plot (or boxplot) represents five numbers

- The box gives the quartiles q_1, q_2, q_3 of the distribution. The middle bar q_2 is thus the median, so 50% of the observations are smaller or larger than this number.
- The length of the whiskers is up to 1.5 times the interquartiles range $q_3 - q_1$ (the whiskers extend until the latest point in the interval, so the largest observation that is smaller than $q_3 + 1.5(q_3 - q_1)$, etc.)

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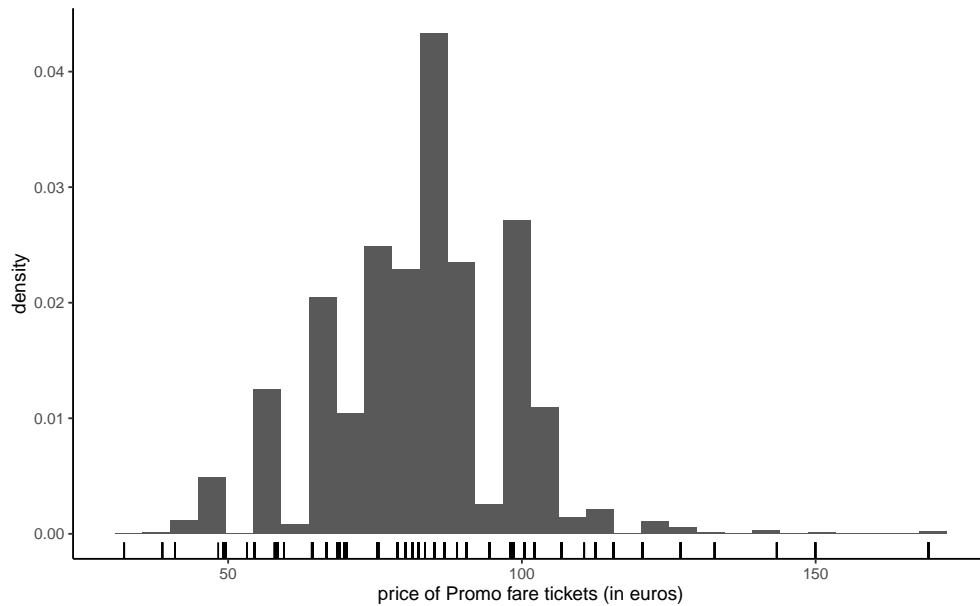


Figure 1.6: Histogram of Promo tickets for Renfe ticket data

- Observations beyond the whiskers are represented by dots or circles, sometimes termed outliers. However, beware of this terminology: the larger the sample size, the more values will fall outside the whiskers. This is a drawback of boxplots, which was conceived at a time where the size of data sets was much smaller than what is current standards.

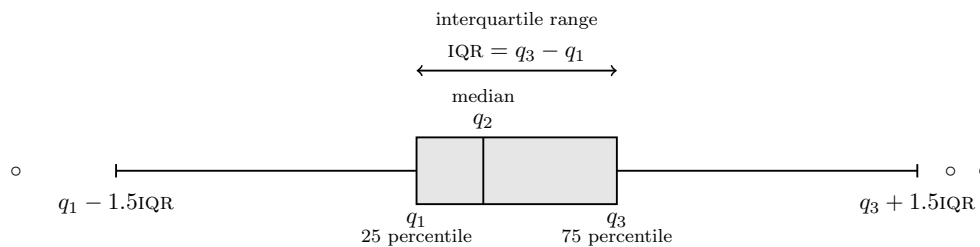


Figure 1.7: Box-and-whiskers plot

We can represent the distribution of a response variable as a function of a categorical variable by drawing a boxplot for each category and laying them side by side. A third variable, categorical, can be added via a color palette, as shown in Figure 1.8.

Scatterplots are used to represent graphically the co-variation between two continuous

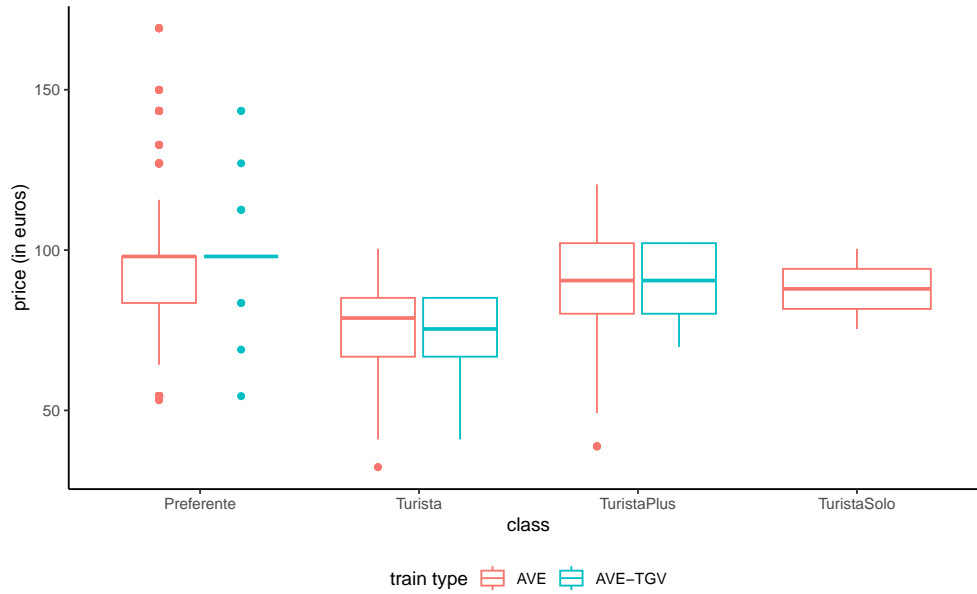


Figure 1.8: Box-and-whiskers plots for Promo fare tickets as a function of class and type for the Renfe tickets data.

variables: each tuple gives the coordinate of the point. If only a handful of large values are visible on the graph, a transformation may be useful: oftentimes, you will encounter graphs where the x - or y -axis is on the log-scale when the underlying variable is positive. If the number of data points is too large, it is hard to distinguish points because they are overlaid: adding transparency, or binning using a two-dimensional histogram with the frequency represented using color are potential solutions. The left panel of Figure 1.9 shows the 100 simulated observations, whereas the right-panel shows a larger sample of 10 000 points using hexagonal binning, an analog of the bivariate density.

Models are (at best) an approximation of the true data generating mechanism and we will want to ensure that our assumptions are reasonable and the quality of the fit decent.

Definition 1.16 (Quantiles-quantiles plots). Quantile-quantile plots are graphical goodness-of-fit diagnostics that are based on the following principle: if Y is a continuous random variable with distribution function F , then the mapping $F(Y) \sim \text{unif}(0, 1)$ yields standard uniform variables. Similarly, the quantile transform applied to a uniform variable provides a mean to simulating samples from F , viz. $F^{-1}(U)$. Consider then a random sample of size n from the uniform distribution ordered from smallest to largest, with $U_{(1)} \leq \dots \leq U_{(n)}$. One can show these ranks have marginally a Beta distribution, $U_{(k)} \sim \text{beta}(k, n + 1 - k)$ with expectation $k/(n + 1)$.

1 Introduction

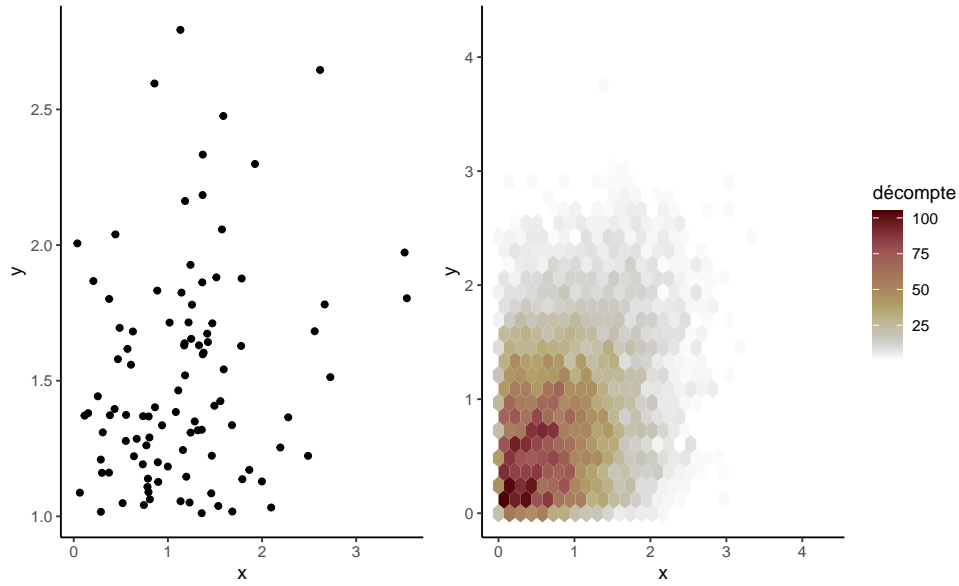


Figure 1.9: Scatterplot (left) and hexagonal heatmap of bidimensional bin counts (right) of simulated data.

In practice, we don't know F and, even if we did, one would need to estimate the parameters. We consider some estimator \hat{F} for the model and apply the inverse transform to an approximate uniform sample $\{i/(n+1)\}_{i=1}^n$. The quantile-quantile plot shows the data as a function of the (first moment) of the transformed order statistics:

- on the x -axis, the theoretical quantiles $\hat{F}^{-1}\{\text{rank}(y_i)/(n+1)\}$
- on the y -axis, the empirical quantiles y_i

If the model is adequate, the ordered values should follow a straight line with unit slope passing through the origin.

Even if we knew the true distribution of the data, the sample variability makes it very difficult to spot if deviations from the model are abnormal or compatible with the model. A simple point estimate with no uncertainty measure can lead to wrong conclusions. As such, we add approximate pointwise or simultaneous confidence intervals. The simplest way to do this is by simulation, by repeating the following steps B times:

1. simulate a sample $\{Y_i^{(b)}\}(i = 1, \dots, n)$ from \hat{F}
2. re-estimate the parameters of F to obtain $\hat{F}_{(b)}$
3. calculate and save the plotting positions $\hat{F}_{(b)}^{-1}\{i/(n+1)\}$.

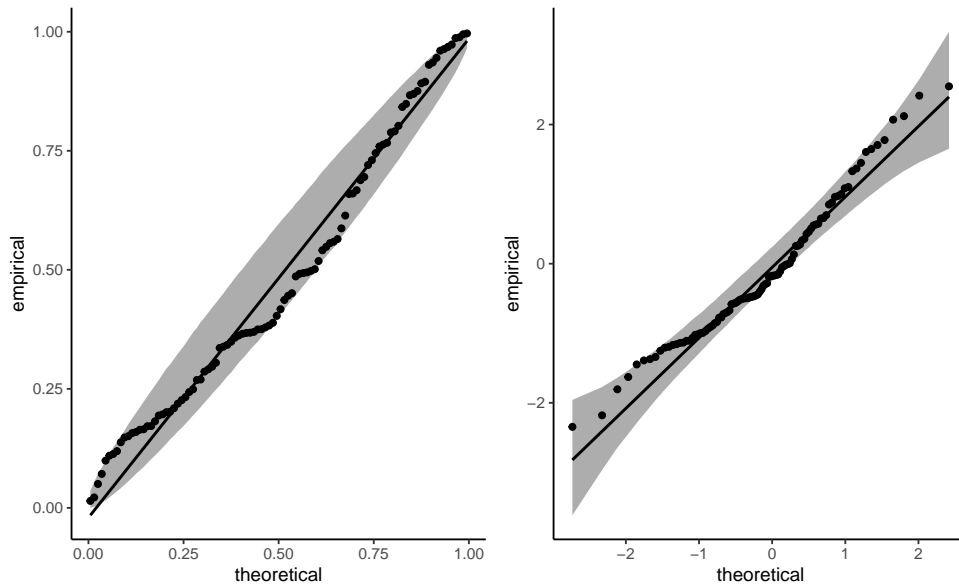


Figure 1.10: Probability-probability plot (left) on uniform margins, and ormal quantile-quantile plot (right) for the same dataset.

The result of this operation is an $n \times B$ matrix of simulated data. We obtain a symmetric $(1 - \alpha)$ confidence interval by keeping the empirical quantile of order $\alpha/2$ and $1 - \alpha/2$ from each row. The number B should be larger than 999, say, and be chosen so that B/α is an integer.

For the pointwise interval, each order statistic from the sample is a statistic and so the probability of any single one falling outside the confidence interval is approximately α . However, order statistics are not independent (they are ordered), so its common to see neighbouring points falling outside of their respective intervals. The intervals shown in Figure 1.10 are pointwise and derived (magically) using a simple function. The uniform order statistics have larger variability as we move away from 0.5, but the uncertainty in the quantile-quantile plot largely depends on F .

Interpretation of quantile-quantile plots requires practice and experience: this post by *Glen_b* on StackOverflow nicely summarizes what can be detected (or not) from them.

1.5 Laws of large numbers

An estimator for a parameter θ is **consistent** if the value obtained as the sample size increases (to infinity) converges to the true value of θ . Mathematically speaking, this translates into convergence in probability, meaning $\hat{\theta} \xrightarrow{\text{Pr}} \theta$. In common language, we say that the probability that $\hat{\theta}$ and θ differ becomes negligible as n gets large.

Consistency is the *a minima* requirement for an estimator: when we collect more information, we should approach the truth. The law of large number states that the sample mean of n (independent) observations with common mean μ , say \bar{Y}_n , converges to μ , denoted $\bar{Y}_n \rightarrow \mu$. Roughly speaking, our approximation becomes less variable and asymptotically unbiased as the sample size (and thus the quantity of information available for the parameter) increases. The law of large number is featured in Monte Carlo experiments: we can approximate the expectation of some (complicated) function $g(x)$ by simulating repeatedly independent draws from Y and calculating the sample mean $n^{-1} \sum_{i=1}^n g(Y_i)$.

If the law of large number tells us what happens in the limit (we get a single numerical value), the result doesn't contain information about the rate of convergence and the uncertainty at finite levels.

1.6 Central Limit Theorem

The central limit theorem gives the approximate large sample distribution of the sample mean. Consider a random sample of size n $\{Y_i\}_{i=1}^n$ of independent random variables with common expectation μ and variance σ^2 . The sample mean $\bar{Y} = n^{-1} \sum_{i=1}^n Y_i$ converges to μ by the law of large number, but we also have that

- the estimator \bar{Y} is centered around μ ,
- the standard error is σ/\sqrt{n} ; the rate of convergence is thus \sqrt{n} . For a sample of size 100, the standard error of the sample mean will be 10 times smaller than that of the underlying random variable.
- the sample mean, once properly scaled, follows approximately a normal distribution

Mathematically, the central limit theorem states $\sqrt{n}(\bar{Y} - \mu) \xrightarrow{d} \text{normal}(0, \sigma^2)$. If n is large (a rule of thumb is $n > 30$, but this depends on the underlying distribution of Y), then $\bar{Y} \sim \text{normal}(\mu, \sigma^2/n)$.

How do we make sense of this result? Let us consider the mean travel time of high speed Spanish trains (AVE) between Madrid and Barcelona that are operated by Renfe.

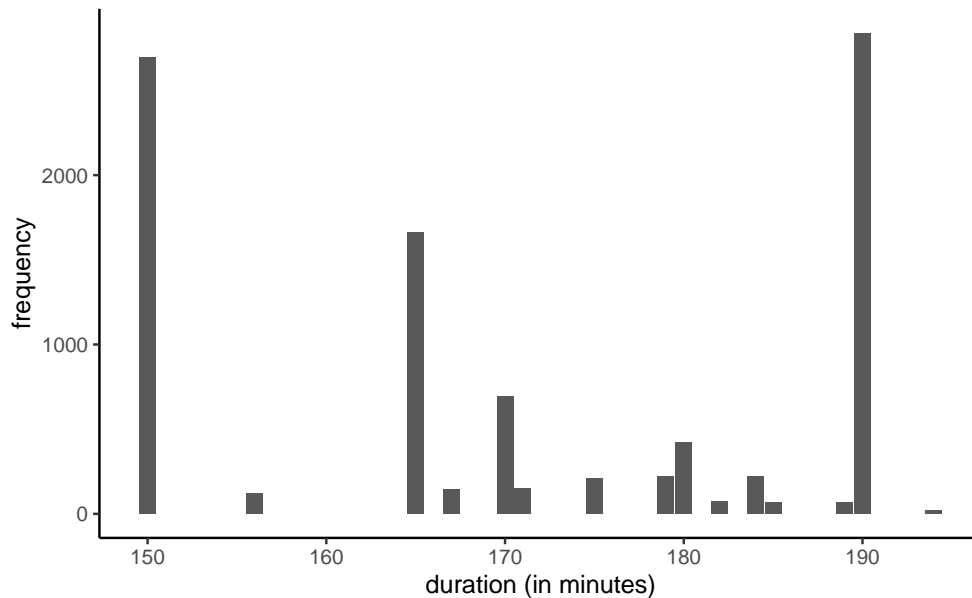


Figure 1.11: Empirical distribution of travel times of high speed trains.

Our exploratory data analysis showed previously that the duration is the one advertised on the ticket: there are only 15 unique travel time. Based on 9603 observations, we estimate the mean travel time to be 170 minutes and 41 seconds. Figure 1.11 shows the empirical distribution of the data.

Consider now samples of size $n = 10$, drawn repeatedly from the population: in the first sample, the sample mean is 169.3 minutes, whereas we get an estimate of 167 minutes in our second, 157.9 minutes in the third, etc.

We draw $B = 1000$ different samples, each of size $n = 5$, from two millions records, and calculate the sample mean in each of them. The top right panel of Figure 1.12 is a histogram of the sample means when $n = 5$, whereas the bottom left panel shows the same thing for $n = 20$. The last graph of Figure 1.12 shows the impact of the increase in sample size: whereas the normal approximation is okay-ish for $n = 5$, it is indistinguishable from the normal approximation for $n = 20$. As n increases and the sample size gets bigger, the quality of the approximation improves and the curve becomes more concentrated around the true mean. Even if the distribution of the travel time is discrete, the mean is approximately normal.

We considered a single distribution in the example, but you could play with other distributions and vary the sample size to see when the central limit theorem kicks in using this applet.

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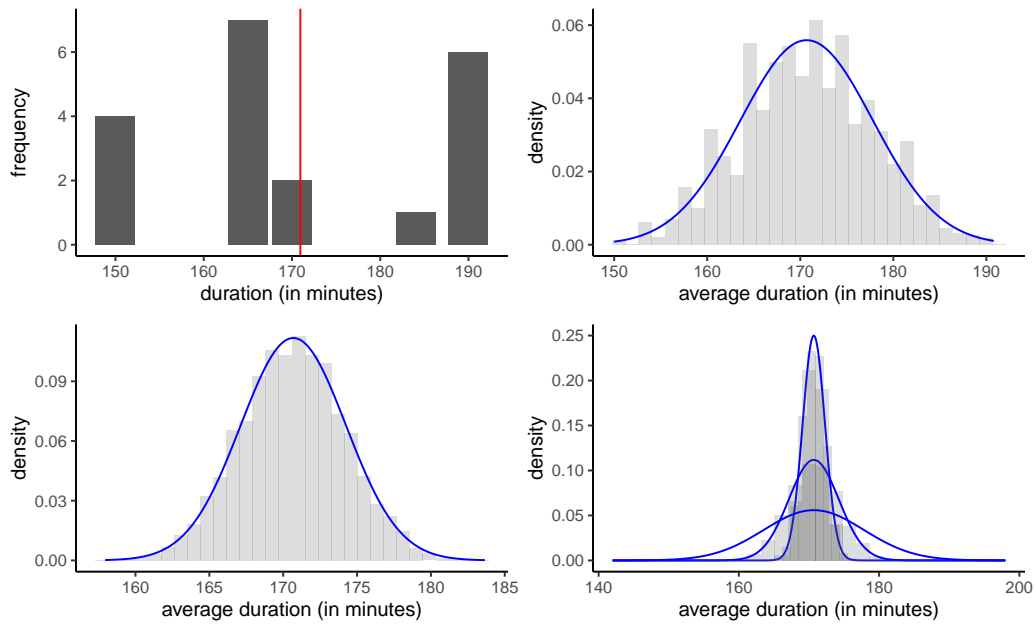


Figure 1.12: Graphical representation of the central limit theorem. The upper left panel shows a sample of 20 observations with its sample mean (vertical red). The three other panels show the histograms of the sample mean from repeated samples of size 5 (top right), 20 (bottom left) and 20, 50 and 100 overlaid, with the density approximation provided by the central limit theorem.

The central limit theorem underlies why scaled test statistics which have sample mean zero and sample variance 1 have a standard null distribution in large sample: this is what guarantees the validity of our inference!

2 Statistical inference

In most applied domains, empirical evidences drive the advancement of the field and data from well designed experiments contribute to the built up of science. In order to draw conclusions in favour or against a theory, researchers turn (often unwillingly) to statistics to back up their claims. This has led to the prevalence of the use of the null hypothesis statistical testing (NHST) framework. One important aspect of the reproducibility crisis is the misuse of p -values in journal articles: falsification of a null hypothesis is not enough to provide substantive findings for a theory.

Because introductory statistics course typically present hypothesis tests without giving much thoughts to the underlying construction principles of such procedures, users often have a reductive view of statistics as a catalogue of pre-determined procedures. To make a culinary analogy, users focus on learning recipes rather than trying to understand the basics of cookery. This chapter focuses on understanding of key ideas related to testing.

! Important

Learning objectives:

- Understanding the role of uncertainty in decision making.
- Understanding the importance of signal-to-noise ratio as a measure of evidence.
- Knowing the basic ingredients of hypothesis testing and being capable of correctly formulating and identifying these components in a paper.
- Correctly interpreting p -values and confidence intervals for a parameter.

The first step of a design is formulating a research question. Generally, this hypothesis will specify potential differences between population characteristics due to some intervention (a treatment) that the researcher wants to quantify. This is the step during which researchers decide on sample size, choice of response variable and metric for the measurement, write down the study plan, etc.

It is important to note that most research questions cannot be answered by simple tools. Researchers wishing to perform innovative methodological research should contact experts and consult with statisticians **before** they collect their data to get information on

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how best to proceed for what they have in mind so as to avoid the risk of making misleading and false claims based on incorrect analysis or data collection.

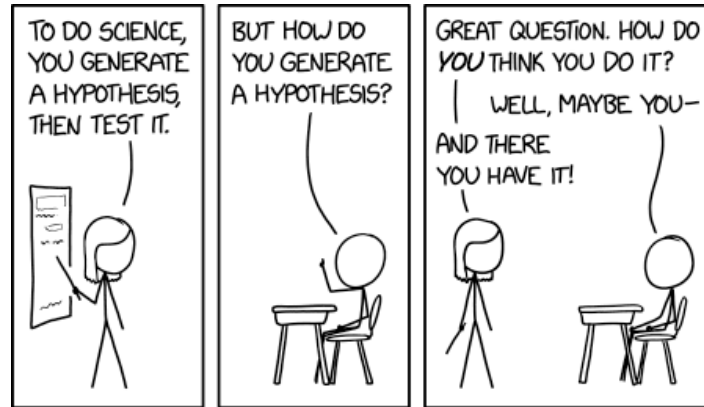


Figure 2.1: xkcd comic 2569 (Hypothesis generation) by Randall Munroe. Alt text: Frazzled scientists are requesting that everyone please stop generating hypotheses for a little bit while they work through the backlog. Cartoon reprinted under the CC BY-NC 2.5 license.

2.1 Sampling variability

Given data, a researcher will be interested in estimating particular characteristics of the population. We can characterize the set of all potential values their measurements can take, together with their frequency, via a distribution.

The purpose of this section is to illustrate how we cannot simply use raw differences between groups to make meaningful comparisons: due to sampling variability, samples will be alike even if they are generated in the same way, but there will be always be differences between their summary statistics. Such differences tend to attenuate (or increase) as we collect more sample. Inherent to this is the fact that as we gather more data (and thus more information) about our target, the portrait becomes more precise. This is ultimately what allows us to draw meaningful conclusions but, in order to do so, we need first to determine what is likely or plausible and could be a stroke of luck, and what is not likely to occur solely due to randomness.

We call numerical summaries of the data **statistics**. Its important to distinguish between procedures/formulas and their numerical values. An **estimator** is a rule or formula used to calculate an estimate of some parameter or quantity of interest based on observed data (like a recipe for cake). Once we have observed data we can actually compute the sample

mean, that is, we have an estimate — an actual value (the cake), which is a single realization and not random. In other words,

- an estimand is our conceptual target, like the population characteristic of interest (population mean).
- an estimator is the procedure or formula telling us how to transform the sample data into a numerical summary that is a proxy of our target.
- an estimate is a number, the numerical value obtained once we apply the formula to observed data.

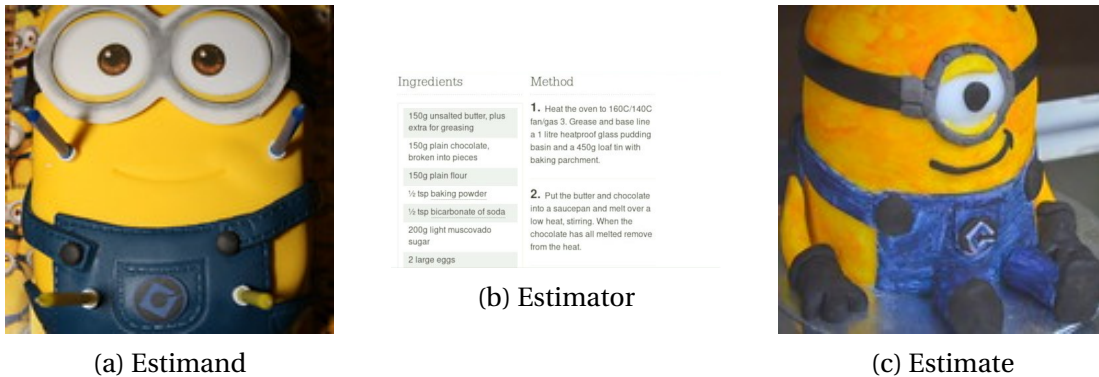


Figure 2.2: Estimand (left), estimator (middle) and estimate (right) illustrated with cakes and based on an original idea of Simon Grund. Cake photos shared under CC BY-NC 2.0 license.

For example, we may use as estimand the population average of Y_1, \dots , say μ . The estimator will be sample mean, i.e., the sum of the elements in the sample divided by the sample size, $\bar{Y} = (Y_1 + \dots + Y_n)/n$. The estimate will be a numerical value, say 4.3.

Because the inputs of the estimator are random, the output is also random and change from one sample to the next: even if you repeat a recipe, you won't get the exact same result every time, as in Figure 2.3.

To illustrate this point, Figure 2.4 shows five simple random samples of size $n = 10$ drawn from an hypothetical population with mean μ and standard deviation σ , along with their sample mean \bar{y} . Because of the sampling variability, the sample means of the subgroups will differ even if they originate from the same distribution. You can view sampling variability as noise: our goal is to extract the signal (typically differences in means) but accounting for spurious results due to the background noise.

The astute eye might even notice that the sample means (thick horizontal segments) are less dispersed around the full black horizontal line representing the population average μ

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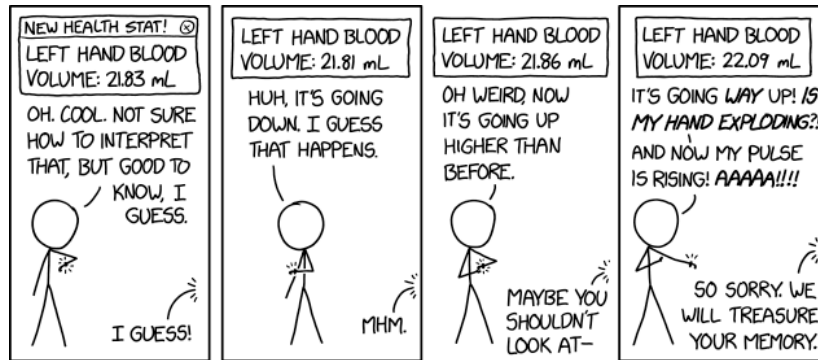


Figure 2.3: xkcd comic 2581 (Health Stats) by Randall Munroe. Alt text: You will live on forever in our hearts, pushing a little extra blood toward our left hands now and then to give them a squeeze. Cartoon reprinted under the CC BY-NC 2.5 license.

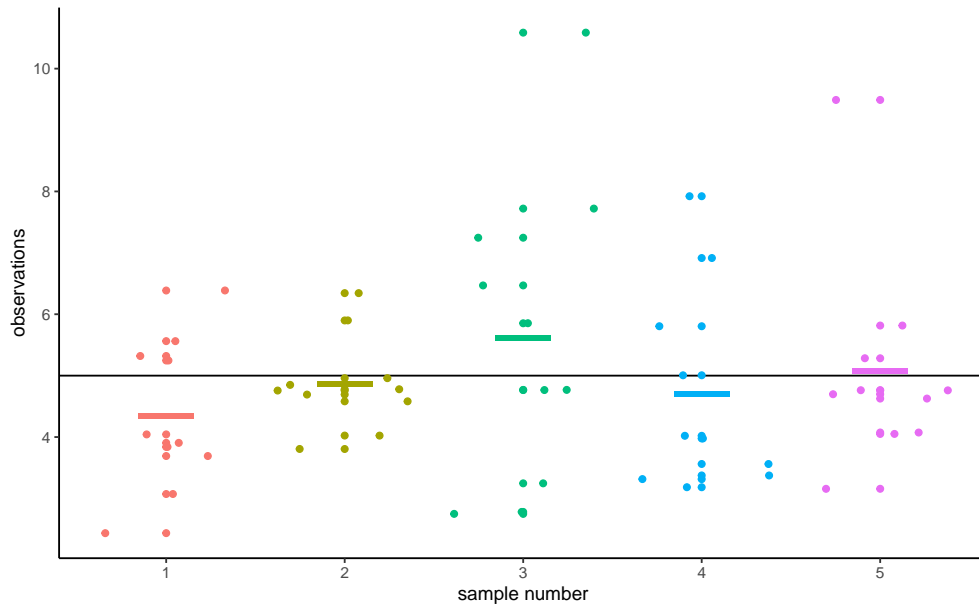


Figure 2.4: Five samples of size $n = 10$ drawn from a common population with mean μ (horizontal line). The colored segments show the sample means of each sample.

than are the individual measurements. This is a fundamental principle of statistics: information accumulates as you get more data.

Values of the sample mean don't tell the whole picture and studying differences in mean

(between groups, or relative to a postulated reference value) is not enough to draw conclusions. In most settings, there is no guarantee that the sample mean will be equal to its true value because it changes from one sample to the next: the only guarantee we have is that it will be on average equal to the population average in repeated samples. Depending on the choice of measurement and variability in the population, there may be considerable differences from one observation to the next and this means the observed difference could be a fluke.

To get an idea of how certain something is, we have to consider the variability of an observation Y_i . This variance of an observation drawn from the population is typically denoted σ^2 and its square root, the standard deviation, by σ .

The standard deviation *of a statistic* is termed **standard error**; it should not be confused with the standard deviation σ of the population from which the sample observations Y_1, \dots, Y_n are drawn. Both standard deviation and standard error are expressed in the same units as the measurements, so are easier to interpret than variance. Since the standard error is a function of the sample size, it is however good practice to report the estimated standard deviation in reports.

Example 2.1 (Sample proportion and uniform draws). To illustrate the concept of sampling variability, we follow the lead of Matthew Crump and consider samples from a uniform distribution on $\{1, 2, \dots, 10\}$ each number in this interval is equally likely to be sampled.

Even if they are drawn from the same population, the 10 samples in Figure 2.5 look quite different. The only thing at play here is the sample variability: since there are $n = 20$ observations in total, there should be on average 10% of the observations in each of the 10 bins, but some bins are empty and others have more counts than expected. This fluctuation is due to randomness, or chance.

How can we thus detect whether what we see is compatible with the model we think generated the data? The key is to collect more observations: the bar height is the sample proportion, an average of 0/1 values with ones indicating that the observation is in the bin and zero otherwise.

Consider now what happens as we increase the sample size: the top panel of Figure 2.6 shows uniform samples for increasing samples size. The scaled bar plot looks more and more like the true underlying distribution (flat, each bin with equal frequency) as the sample size increases. The sample distribution of points is nearly indistinguishable from the theoretical one (straight line) when $n = 10000$.¹ The bottom panel, on the other hand,

¹The formula shows that the standard error decreases by a tenfold every time the sample size increases by a factor 100.

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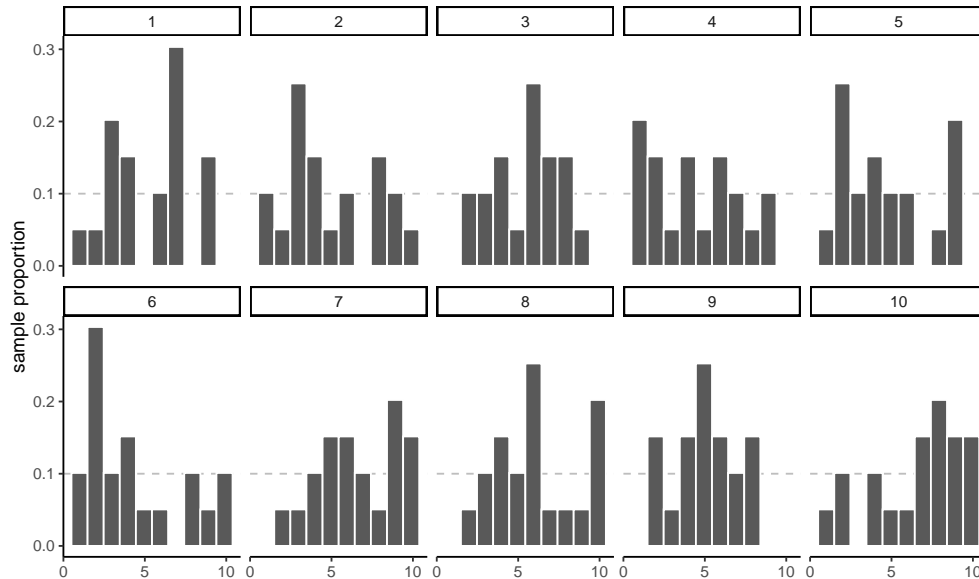


Figure 2.5: Histograms for 10 random samples of size $n = 20$ from a discrete uniform distribution.

isn't from a uniform distribution and larger samples come closer to the population distribution. We couldn't have spotted this difference in the first two plots, since the sampling variability is too important; there, the lack of data in some bins could have been attributed to chance, as they are comparable with the graph for data that are truly uniform. This is in line with most practical applications, in which the limited sample size restricts our capacity to disentangle real differences from sampling variability. We must embrace this uncertainty: in the next section, we outline how hypothesis testing helps us disentangle the signal from the noise.

2.2 Hypothesis testing

An **hypothesis test** is a binary decision rule used to evaluate the statistical evidence provided by a sample to make a decision regarding the underlying population. The main steps involved are:

- define the model parameters
- formulate the alternative and null hypothesis
- choose and calculate the test statistic

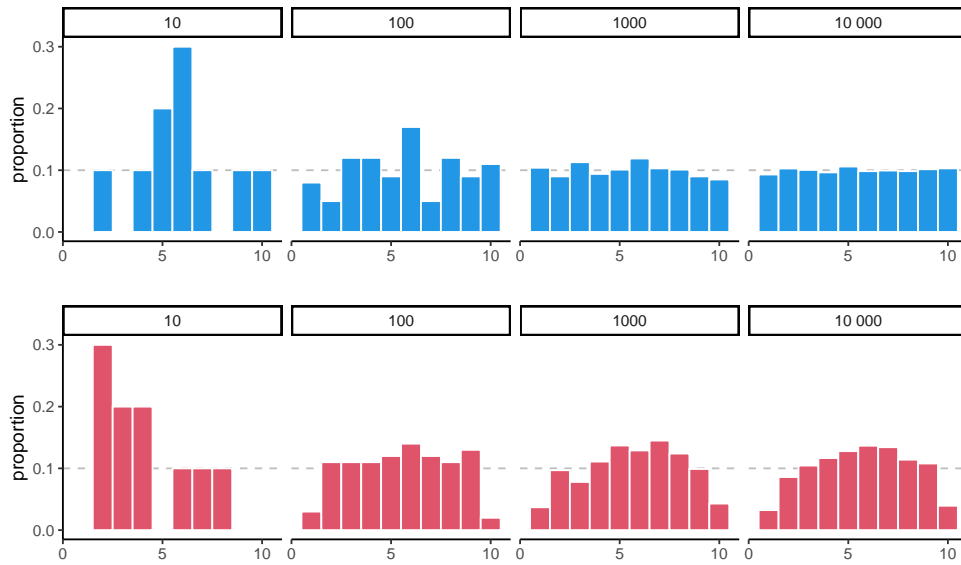


Figure 2.6: Bar plots of data from a uniform distribution (top) and non-uniform (bottom) with increasing sample sizes of 10, 100, 1000 and 10 000 (from left to right).

- obtain the null distribution describing the behaviour of the test statistic under \mathcal{H}_0
- calculate the p -value
- conclude (reject or fail to reject \mathcal{H}_0) in the context of the problem.

A good analogy for hypothesis tests is a trial for murder on which you are appointed juror.

- The judge lets you choose between two mutually exclusive outcome, guilty or not guilty, based on the evidence presented in court.
- The presumption of innocence applies and evidences are judged under this optic: are evidence remotely plausible if the person was innocent? The burden of the proof lies with the prosecution to avoid as much as possible judicial errors. The null hypothesis \mathcal{H}_0 is *not guilty*, whereas the alternative \mathcal{H}_a is *guilty*. If there is a reasonable doubt, the verdict of the trial will be not guilty.
- The test statistic (and the choice of test) represents the summary of the proof. The more overwhelming the evidence, the higher the chance the accused will be declared guilty. The prosecutor chooses the proof so as to best outline this: the choice of evidence (statistic) ultimately will maximise the evidence, which parallels the power of the test.
- The final step is the verdict. This is a binary decision, guilty or not guilty. For an

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hypothesis test performed at level α , one would reject (guilty) if the p -value is less than α .

The above description provides some heuristic, but lacks crucial details.

2.3 Hypothesis

In statistical tests we have two hypotheses: the null hypothesis (\mathcal{H}_0) and the alternative hypothesis (\mathcal{H}_1). Usually, the null hypothesis is the ‘status quo’ and the alternative is what we’re really interested in testing. A statistical hypothesis test allows us to decide whether or not our data provides enough evidence to reject \mathcal{H}_0 in favour of \mathcal{H}_1 , subject to some pre-specified risk of error. Usually, hypothesis tests involve a parameter, say θ , which characterizes the underlying distribution at the population level and whose value is unknown. A two-sided hypothesis test regarding a parameter θ has the form

$$\mathcal{H}_0 : \theta = \theta_0 \quad \text{versus} \quad \mathcal{H}_a : \theta \neq \theta_0.$$

We are testing whether or not θ is precisely equal to the value θ_0 . The hypotheses are a statistical representation of our research question.

A common example of two-sided test is one for the regression coefficient β_j associated to an explanatory variable X_j , for which the null and alternative hypothesis are

$$\mathcal{H}_0 : \beta_j = \beta_j^0 \quad \text{versus} \quad \mathcal{H}_a : \beta_j \neq \beta_j^0,$$

where β_j^0 is some value that reflects the research question of interest. For example, if $\beta_j^0 = 0$, the underlying question is: is covariate X_j impacting the response Y linearly once other variables have been taken into account?

Note that we can impose direction in the hypotheses and consider alternatives of the form $\mathcal{H}_a : \theta > \theta_0$ or $\mathcal{H}_a : \theta < \theta_0$.

2.4 Test statistic

A test statistic T is a function of the data that summarise the information contained in the sample for θ . The form of the test statistic is chosen such that we know its underlying distribution under \mathcal{H}_0 , that is, the potential values taken by T and their relative probability if \mathcal{H}_0 is true. Indeed, T is a random variable and its value change from one sample to the

2.5 Null distribution and p-value

next. This allows us to determine what values of T are likely if \mathcal{H}_0 is true. Many statistics we will consider are **Wald statistic**, of the form

$$T = \frac{\hat{\theta} - \theta_0}{\text{se}(\hat{\theta})}$$

where $\hat{\theta}$ is an estimator of θ , θ_0 is the postulated value of the parameter and $\text{se}(\hat{\theta})$ is an estimator of the standard deviation of the test statistic $\hat{\theta}$.

For example, to test whether the mean of a population is zero, we set

$$\mathcal{H}_0 : \mu = 0, \quad \mathcal{H}_a : \mu \neq 0,$$

and the Wald statistic is

$$T = \frac{\bar{X} - 0}{S_n / \sqrt{n}}$$

where \bar{X} is the sample mean of X_1, \dots, X_n ,

$$\bar{X} = \frac{1}{n} \sum_{i=1}^n X_i = \frac{X_1 + \dots + X_n}{n}$$

and the standard error (of the mean) \bar{X} is S_n / \sqrt{n} ; the sample variance S_n is an estimator of the standard deviation σ ,

$$S_n^2 = \frac{1}{n-1} \sum_{i=1}^n (X_i - \bar{X})^2.$$

2.5 Null distribution and p-value

The p -value allows us to decide whether the observed value of the test statistic T is plausible under \mathcal{H}_0 . Specifically, the p -value is the probability that the test statistic is equal or more extreme to the estimate computed from the data, assuming \mathcal{H}_0 is true. Suppose that based on a random sample Y_1, \dots, Y_n we obtain a statistic whose value $T = t$. For a two-sided test $\mathcal{H}_0 : \theta = \theta_0$ vs. $\mathcal{H}_a : \theta \neq \theta_0$, the p -value is $\Pr_0(|T| \geq |t|)$.²

How do we determine the null distribution given that the true data generating mechanism is unknown to us? We ask a statistician! In simple cases, it might be possible to enumerate all possible outcomes and thus quantify the degree of outlyingness of our observed statistic. In more general settings, we can resort to simulations or to probability theory: the

²If the distribution of T is symmetric around zero, the p -value reduces to $p = 2 \times \Pr_0(T \geq |t|)$.

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central limit theorem says that the sample mean behaves like a normal random variable with mean μ and standard deviation σ/\sqrt{n} for n large enough. The central limit theorem has broader applications since most statistics can be viewed as some form of average or transformation thereof, a fact used to derive benchmarks for most commonly used tests. Most software use these approximations as proxy by default: the normal, Student's t , χ^2 and F distributions are the reference distributions that arise the most often.

Figure 2.7 shows the distribution of p -values for two scenarios: one in which there are no differences and the null is true, the other under an alternative. The probability of rejection is obtained by calculating the area under the density curve between zero and $\alpha = 0.1$, here 0.1 on the left. Under the null, the model is calibrated and the distribution of p -values is uniform (i.e., a flat rectangle of height 1), meaning all values in the unit interval are equally likely. Under the alternative (right), small p -values are more likely to be observed.

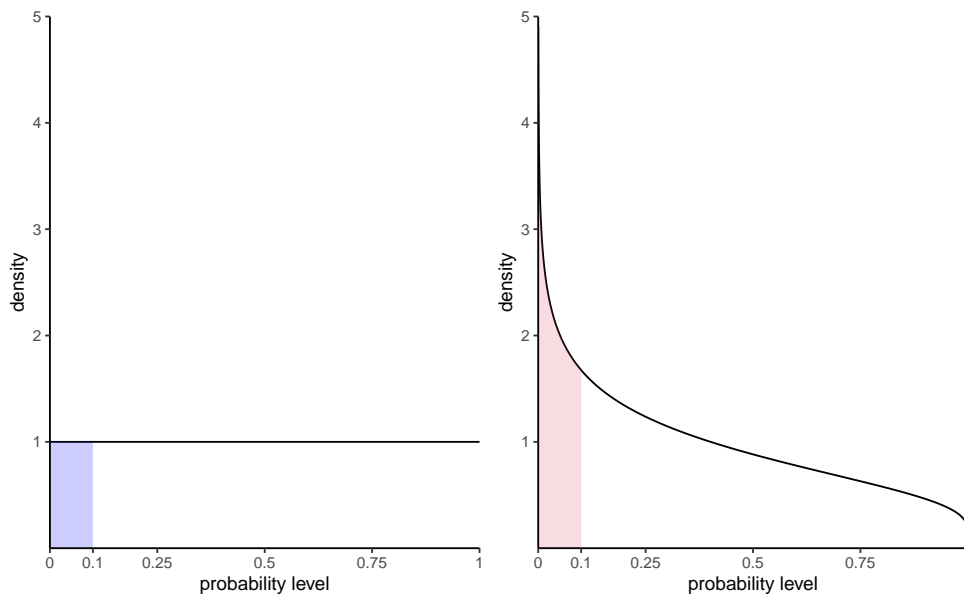


Figure 2.7: Density of p -values under the null hypothesis (left) and under an alternative with a signal-to-noise ratio of 0.5 (right).

There are generally three ways of obtaining null distributions for assessing the degree of evidence against the null hypothesis

- exact calculations
- large sample theory (aka 'asymptotics' in statistical lingo)
- simulation

While desirable, the first method is only applicable in simple cases (such as counting the probability of getting two six if you throw two fair die). The second method is most commonly used due to its generality and ease of use (particularly in older times where computing power was scarce), but fares poorly with small sample sizes (where ‘too small’ is context and test-dependent). The last approach can be used to approximate the null distribution in many scenarios, but adds a layer of randomness and the extra computations costs sometimes are not worth it.

Consider the example of a two-sided test involving the population mean $\mathcal{H}_0 : \mu = 0$ against the alternative $\mathcal{H}_1 : \mu \neq 0$. Assuming the random sample comes from a normal (population) $\text{normal}(\mu, \sigma^2)$, it can be shown that if \mathcal{H}_0 is true (that is, if $\mu = 0$), the test statistic

$$T = \frac{\bar{X}}{S/\sqrt{n}}$$

follows a Student- t distribution with $n - 1$ degrees of freedom. This allows us to calculate the p -value (either from a table, or using some statistical software). By virtue of the symmetry, the p -value is $P = 2 \times \Pr(T > |t|)$, where $T \sim \text{Student}(n - 1)$.

2.6 Confidence intervals

A **confidence interval** is an alternative way to present the conclusions of an hypothesis test performed at significance level α . It is often combined with a point estimator $\hat{\theta}$ plus or minus a margin of error designed to give an indication of the variability of the estimation procedure. Wald-based $(1 - \alpha)$ confidence intervals for a scalar parameter θ are of the form

$$[\hat{\theta} + q_{\alpha/2} \text{se}(\hat{\theta}), \hat{\theta} + q_{1-\alpha/2} \times \text{se}(\hat{\theta})]$$

where $q_{\alpha/2}$ is the $\alpha/2$ quantile of the null distribution of the Wald statistic W ,

$$W = \frac{\hat{\theta} - \theta}{\text{se}(\hat{\theta})},$$

and where θ represents the postulated value for the fixed, but unknown value of the parameter. The critical values for a symmetric interval, chosen so that the probability of being more extreme is α , are the $\alpha/2$ and $1 - \alpha/2$ quantiles of the null distribution.

For example, for a random sample X_1, \dots, X_n from a normal distribution $\text{normal}(\mu, \sigma)$, the $(1 - \alpha)$ confidence interval for the population mean μ is

$$\bar{X} \pm t_{n-1, \alpha/2} \frac{S}{\sqrt{n}}$$

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where $t_{n-1, \alpha/2}$ is the $1 - \alpha/2$ quantile of a Student- t distribution with $n - 1$ degrees of freedom.

The bounds of the confidence intervals are random variables, since both estimators of the parameter and its standard error, $\hat{\theta}$ and $\text{se}(\hat{\theta})$, are random: their values will vary from one sample to the next. Before the interval is calculated, there is a $1 - \alpha$ probability that θ is contained in the **random** interval $(\hat{\theta} - q_{\alpha/2} \text{se}(\hat{\theta}), \hat{\theta} + q_{\alpha/2} \text{se}(\hat{\theta}))$, where $\hat{\theta}$ denotes the estimator. Once we obtain a sample and calculate the confidence interval, there is no more notion of probability: the true value of the parameter θ is either in the confidence interval or not. We can interpret confidence intervals as follows: if we were to repeat the experiment multiple times, and calculate a $1 - \alpha$ confidence interval each time, then roughly $1 - \alpha$ of the calculated confidence intervals would contain the true value of θ in repeated samples (in the same way, if you flip a coin, there is roughly a 50-50 chance of getting heads or tails, but any outcome will be either). Our confidence is in the *procedure* we use to calculate confidence intervals and not in the actual values we obtain from a sample.

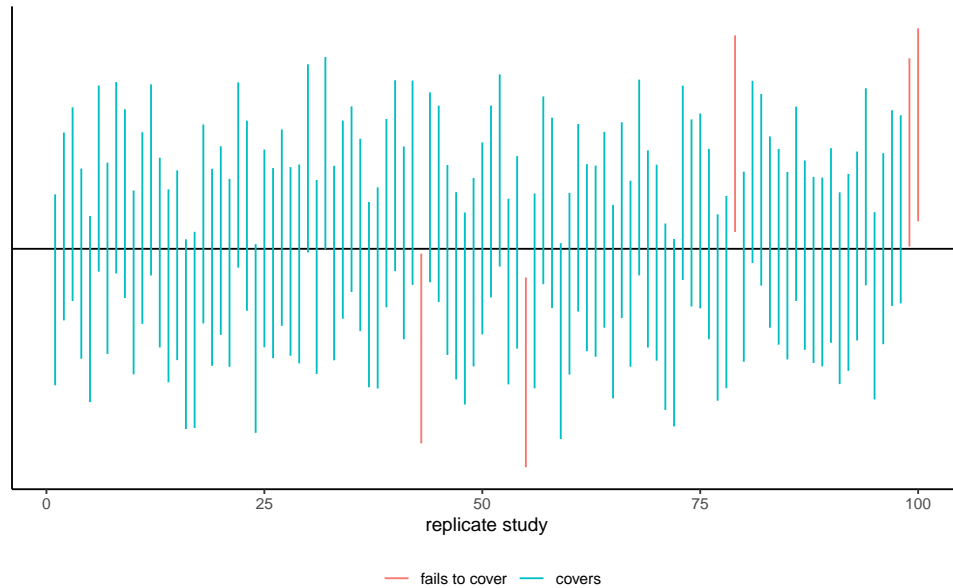


Figure 2.8: 95% confidence intervals for the mean of a standard normal population for 100 random samples. On average, 5% of these intervals fail to include the true mean value of zero (in red).

If we are only interested in the binary decision rule reject/fail to reject \mathcal{H}_0 , the confidence interval is equivalent to a p -value since it leads to the same conclusion. Whereas the $1 - \alpha$ confidence interval gives the set of all values for which the test statistic doesn't provide enough evidence to reject \mathcal{H}_0 at level α , the p -value gives the probability under the null of

obtaining a result more extreme than the postulated value and so is more precise for this particular value. If the p -value is smaller than α , our null value θ will be outside of the confidence interval and vice-versa.

2.7 Conclusion

The p -value allows us to make a decision about the null hypothesis. If \mathcal{H}_0 is true, the p -value follows a uniform distribution. Thus, if the p -value is small, this means observing an outcome more extreme than $T = t$ is unlikely, and so we're inclined to think that \mathcal{H}_0 is not true. There's always some underlying risk that we're making a mistake when we make a decision. In statistic, there are two type of errors:

- type I error: we reject \mathcal{H}_0 when \mathcal{H}_0 is true,
- type II error: we fail to reject \mathcal{H}_0 when \mathcal{H}_0 is false.

These hypothesis are not judged equally: we seek to avoid error of type I (judicial errors, corresponding to condemning an innocent). To prevent this, we fix the level of the test, α , which captures our tolerance to the risk of committing a type I error: the higher the level of the test α , the more often we will reject the null hypothesis when the latter is true. The value of $\alpha \in (0, 1)$ is the probability of rejecting \mathcal{H}_0 when \mathcal{H}_0 is in fact true,

$$\alpha = \Pr_0(\text{reject } \mathcal{H}_0).$$

where the subscript \Pr_0 indicates the probability under the null model. The level α is fixed beforehand, typically 1%, 5% or 10%. Keep in mind that the probability of type I error is α only if the null model for \mathcal{H}_0 is correct (sic) and correspond to the data generating mechanism.

The focus on type I error is best understood by thinking about medical trial: you need to prove a new cure is better than existing alternatives drugs or placebo, to avoid extra costs or harming patients (think of Didier Raoult and his unsubstantiated claims that hydrochloroquine, an antipaludean drug, should be recommended treatment against Covid19).

Decision \ true model	\mathcal{H}_0	\mathcal{H}_a
fail to reject \mathcal{H}_0	✓	type II error
reject \mathcal{H}_0	type I error	✓

To make a decision, we compare our p -value P with the level of the test α :

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- if $P < \alpha$, we reject \mathcal{H}_0 ;
- if $P \geq \alpha$, we fail to reject \mathcal{H}_0 .

Do not mix up level of the test (probability fixed beforehand by the researcher) and the p -value. If you do a test at level 5%, the probability of type I error is by definition α and does not depend on the p -value. The latter is conditional probability of observing a more extreme likelihood given the null distribution \mathcal{H}_0 is true.

Caution

The American Statistical Association (ASA) published a list of principles guiding (mis)interpretation of p -values, some of which are reproduced below:

- (2) P -values do not measure the probability that the studied hypothesis is true.
- (3) Scientific conclusions and business or policy decisions should not be based only on whether a p -value passes a specific threshold.
- (4) P -values and related analyses should not be reported selectively.
- (5) p -value, or statistical significance, does not measure the size of an effect or the importance of a result.

2.8 Power

There are two sides to an hypothesis test: either we want to show it is not unreasonable to assume the null hypothesis, or else we want to show beyond reasonable doubt that a difference or effect is significative: for example, one could wish to demonstrate that a new website design (alternative hypothesis) leads to a significant increase in sales relative to the status quo. Our ability to detect these improvements and make discoveries depends on the power of the test: the larger the power, the greater our ability to reject \mathcal{H}_0 when the latter is false.

Failing to reject \mathcal{H}_0 when \mathcal{H}_a is true corresponds to the definition of type II error, the probability of which is $1 - \text{power}$, say. The **power of a test** is the probability of rejecting \mathcal{H}_0 when \mathcal{H}_0 is false, i.e.,

$$\Pr_a(\text{reject } \mathcal{H}_0),$$

i.e., the probability under the alternative model of falling in the rejection region. Depending on the alternative models, it is more or less easy to detect that the null hypothesis is false and reject in favor of an alternative.

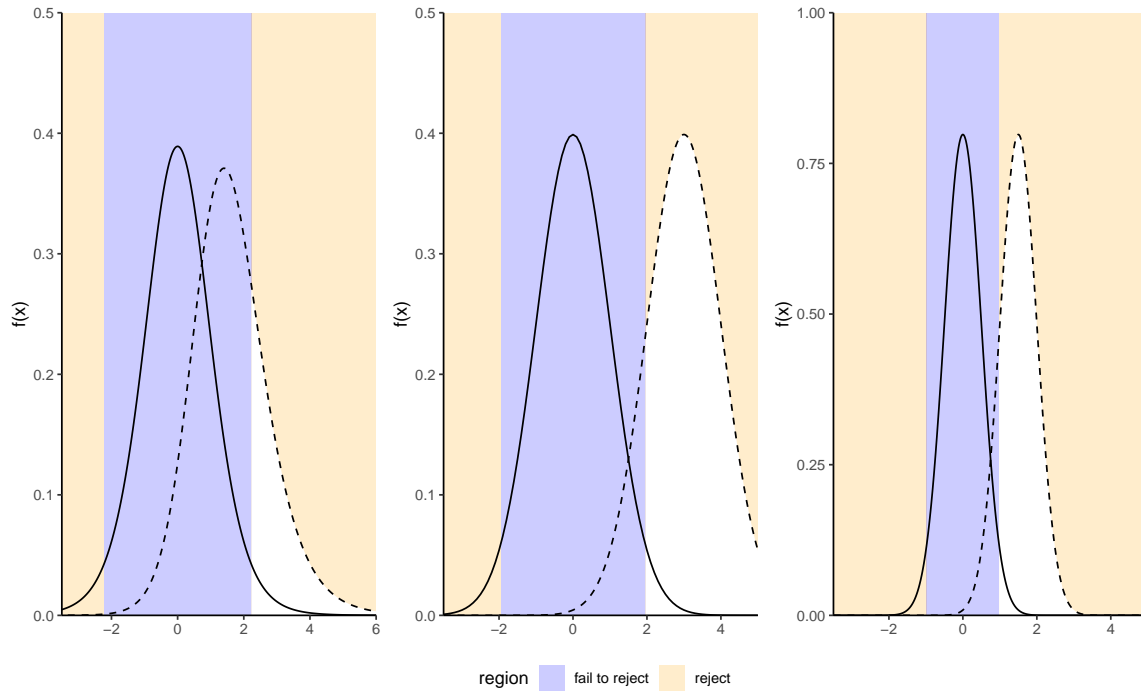


Figure 2.9: Comparison between null distribution (full curve) and a specific alternative for a t -test (dashed line). The power corresponds to the area under the curve of the density of the alternative distribution which is in the rejection area (in white). The middle panel shows an increase in power due to an increase in the mean difference, whereas the right panel shows the change due to a decrease in variability of increase in the sample size.

We want a test to have high power, i.e., that the power should be as close to 1 as possible. Minimally, the power of the test should be α because we reject the null hypothesis α fraction of the time even when \mathcal{H}_0 is true. Power depends on many criteria, notably

- the effect size: the bigger the difference between the postulated value for θ_0 under \mathcal{H}_0 and the observed behavior, the easier it is to detect it, as in the middle panel of Figure 2.9;
- variability: the less noisy your data, the easier it is to detect differences between the curves (big differences are easier to spot, as the right panel of Figure 2.9 shows);

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- the sample size: the more observation, the higher our ability to detect significant differences because the standard error decreases with sample size n at a rate (typically) of $n^{-1/2}$. The null distribution also becomes more concentrated as the sample size increases.
- the choice of test statistic: for example, rank-based statistics discard information about the actual values and care only about relative ranking. Resulting tests are less powerful, but are typically more robust to model misspecification and outliers. The statistics we will choose are standard and amongst the most powerful: as such, we won't dwell on this factor.

To calculate the power of a test, we need to single out a specific alternative hypothesis. In very special cases, analytic derivations are possible but typically we compute the power of a test through Monte Carlo methods. For a given alternative, we simulate repeatedly samples from the model, compute the test statistic on these new samples and the associated p -values based on the postulated null hypothesis. We can then calculate the proportion of tests that lead to a rejection of the null hypothesis at level α , namely the percentage of p -values smaller than α .

2.9 Examples

Example 2.2 (Gender inequality and permutation tests). We consider data from Rosen and Jerdee (1974), who look at sex role stereotypes and their impacts on promotion and opportunities for women candidates. The experiment took place in 1972 and the experimental units, which consisted of 95 male bank supervisors, were submitted to various memorandums and asked to provide ratings or decisions based on the information provided.

We are interested in Experiment 1 related to promotion of employees: managers were requested to decide on whether or not to promote an employee to become branch manager based on recommendations and ratings on potential for customer and employee relations.

The authors' intervention focused on the description of the nature (complexity) of the manager's job (either simple or complex) and the sex of the candidate (male or female): all files were otherwise similar.

We consider for simplicity only sex as a factor and aggregate over job for the $n = 93$ replies. Table 2.2 shows the counts for each possibility.

Table 2.2: Promotion recommendation to branch manager based on sex of the applicant.

	male	female
promote	32	19

The null hypothesis of interest here that sex has no impact, so the probability of promotion is the same for men and women. Let p_m and p_w denote these respective probabilities; we can thus write mathematically the null hypothesis as $\mathcal{H}_0 : p_m = p_w$ against the alternative $\mathcal{H}_a : p_m \neq p_w$.

The test statistic typically employed for contingency tables is a chi-square test³, which compares the overall proportions of promoted to that in for each subgroup. The sample proportion for male is $32/42 = \sim 76\%$, compared to $19/49$ or $\sim 49\%$ for female. While it seems that this difference of 16% is large, it could be spurious: the standard error for the sample proportions is roughly 3.2% for male and 3.4% for female.

If there was no discrimination based on sex, we would expect the proportion of people promoted to be the same overall; this is $51/93 = 0.55$ for the pooled sample. We could simply do a test for the mean difference, but rely instead on the Pearson contingency X_p^2 (aka chi-square) test, which compares the expected counts (based on equal promotion rates) to the observed counts, suitably standardized. If the discrepancy is large between expected and observed, then this casts doubt on the validity of the null hypothesis.

If the counts of each cell are large, the null distribution of the chi-square test is well approximated by a χ^2 distribution. The output of the test includes the value of the statistic, 10.79, the degrees of freedom of the χ^2 approximation and the p -value, which gives the probability that a random draw from a χ^2_1 distribution is larger than the observed test statistic **assuming the null hypothesis is true**. The p -value is very small, 0.001, which means such a result is quite unlikely to happen by chance if there was no sex-discrimination.

Another alternative to obtain a benchmark to assess the outlyingness of the observed odds ratio is to use simulations: permutation tests are well illustrated by Jared Wilber. Consider a database containing the raw data with 93 rows, one for each manager, with for each an indicator of `action` and the `sex` of the hypothetical employee presented in the task.

Table 2.3: First five rows of the database in long format for experiment 1 of Rosen and Jerdee.

action	sex
promote	male
hold file	female
promote	male

³If you have taken advanced modelling courses, this is a score test obtained by fitting a Poisson regression with `sex` and `action` as covariates; the null hypothesis corresponding to lack of interaction term between the two.

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hold file	female
hold file	male

Under the null hypothesis, sex has no incidence on the action of the manager. This means we could get an idea of the “what-if” world by shuffling the sex labels repeatedly. Thus, we could obtain a benchmark by repeating the following steps multiple times:

1. permute the labels for `sex`,
2. recreate a contingency table by aggregating counts,
3. calculate a test statistic for the simulated table.

As test statistic, we use odds ratio: the odds of an event is the ratio of the number of success over failure: in our example, this would be the number of promoted over held files. The odds of promotion for male is $32/12$, whereas that of female is $19/30$. The odds ratio for male versus female is thus $OR = (32/12)/(19/30) = 4.21$. Under the null hypothesis, \mathcal{H}_0 : $OR = 1$ (same probability of being promoted) (why?)

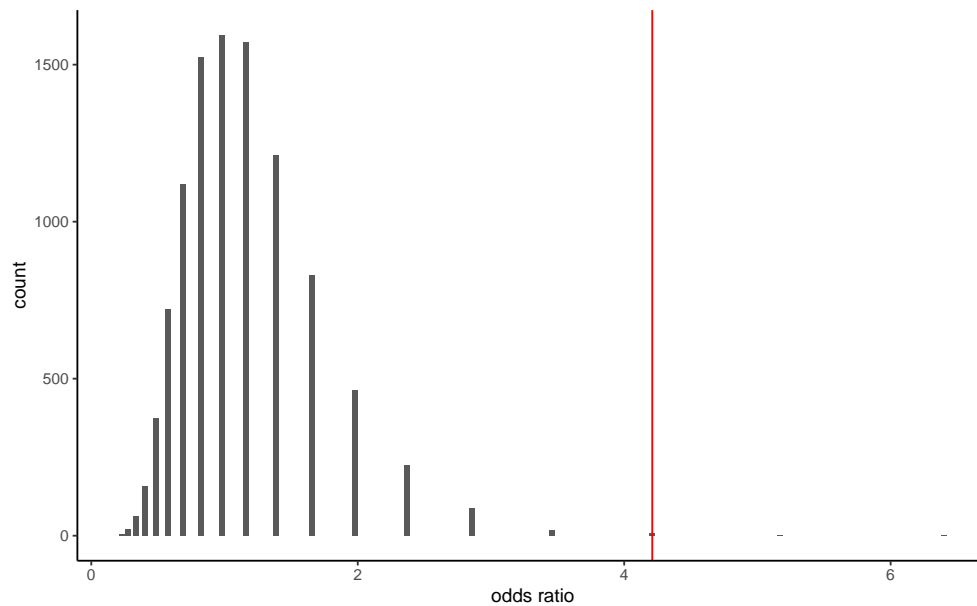


Figure 2.10: Histogram of the simulated null distribution of the odds ratio statistic obtained using a permutation test; the vertical red line indicates the sample odds ratio.

The histogram in Figure 2.10 shows the distribution of the odds ratio based on 10 000 permutations. Reassuringly, we again get roughly the same approximate p -value, here 0.002.⁴

The article concluded (in light of the above and further experiments)

Results confirmed the hypothesis that male administrators tend to discriminate against female employees in personnel decisions involving promotion, development, and supervision.

Recap

- Model parameters: probability of promotion for men and women, respectively p_m and p_w .
- Hypotheses: no discrimination based on gender, meaning equal probability of promotion (null hypothesis $\mathcal{H}_0 : p_m = p_w$, versus alternative hypothesis $\mathcal{H}_a : p_m \neq p_w$).
- Test statistic: (1) chi-square test for contingency tables and (2) odds ratio.
- p -value: (1) .0010 and (2) .0024 based on permutation test.
- Conclusion: reject null hypothesis, as there is evidence of a gender-discrimination with different probability of promotion for men and women.

Following the APA guidelines, the χ^2 statistic would be reported as $\chi^2(1, n = 93) = 10.79$, $p = .001$ along with counts and sample proportions.

Example 2.3 (“The Surprise of Reaching Out”). Liu et al. (2023) studies social interactions and the impact of surprise on people reaching out if this contact is unexpected. Experiment 1 focuses on questionnaires where the experimental condition is the perceived appreciation of reaching out to someone (vs being reached to). The study used a questionnaire administered to 200 American adults recruited on the Prolific Academic platform. The response index consists of the average of four questions measured on a Likert scale ranging from 1 to 7, with higher values indicating higher appreciation.

We can begin by inspecting summary statistics for the sociodemographic variables (gender and age) to assess whether the sample is representative of the general population as a whole. The proportion of other (including non-binary people) is much higher than that of the general census, and the population skews quite young according to Table 2.4.

Table 2.4: Summary statistics of the age of participants, and counts per gender

gender	min	max	mean	n
male	18	78	32.0	105

⁴The p -value obtained for the permutation test would change from one run to the next since its input is random. However, the precision of the proportion statistic is sufficient for decision making purposes.

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female	19	68	36.5	92
other	24	30	27.7	3

Table 2.5: Mean ratings, standard deviation and number of participants per experimental condition.

role	mean	sd	n
initiator	5.50	1.28	103
responder	5.87	1.27	97

Since there are only two groups, initiator and responder, we are dealing with a pairwise comparison. The logical test one could use is a two sample t -test, or a variant thereof. Using Welch two sample t -test statistic, both group average and standard deviation are estimated using the data provided.

The software returns $t(197.52) = -2.05$, $p = .041$, which leads to the rejection of the null hypothesis of no difference in appreciation depending on the role of the individual (initiator or responder). The estimated mean difference is $\Delta M = -0.37$, 95% CI $[-0.73, -0.01]$; since 0 is not included in the confidence interval, we also reject the null hypothesis at level 5%. The estimate suggests that initiators underestimate the appreciation of reaching out.⁵

Recap

- Model parameters: average expected appreciation score μ_i and μ_r of initiators and responder, respectively
- Hypothesis: expected appreciation score is the same for initiator and responders, $\mathcal{H}_0 : \mu_i = \mu_r$ against alternative $\mathcal{H}_a : \mu_i \neq \mu_r$ that they are different.
- Test statistic: Welch two sample t -test
- p -value: 0.041
- Conclusion: reject the null hypothesis, average appreciation score differs depending on the role

Example 2.4 (Virtual communication curbs creative idea generation). A Nature study performed an experiment to see how virtual communications teamwork by comparing the output both in terms of ideas generated during a brainstorming session by pairs and of the quality of ideas, as measured by external referees. The sample consisted of 301 pairs of participants who interacted via either videoconference or face-to-face.

⁵ Assuming that the variance of each subgroup were equal, we could have used a two-sample t -test instead. The difference in the conclusion is immaterial, with a nearly equal p -value.

The authors compared the number of creative ideas, a subset of the ideas generated with creativity score above average. The mean number of the number of creative ideas for face-to-face 7.92 ideas (sd 3.40) relative to videoconferencing 6.73 ideas (sd 3.27).

Brucks and Levav (2022) used a negative binomial regression model: in their model, the expected number creative ideas generated is

$$E(\text{ncreative}) = \exp(\beta_0 + \beta_1 \text{video})$$

where $\text{video} = 0$ if the pair are in the same room and $\text{video} = 1$ if they interact instead via videoconferencing.

The mean number of ideas for videoconferencing is thus $\exp(\beta_1)$ times that of the face-to-face: the estimate of the multiplicative factor is $\exp(\beta_1)$ is 0.85 95% CI [0.77, 0.94].

No difference between experimental conditions translates into the null hypothesis as $\mathcal{H}_0 : \beta_1 = 0$ vs $\mathcal{H}_0 : \beta_1 \neq 0$ or equivalently $\mathcal{H}_0 : \exp(\beta_1) = 1$. The likelihood ratio test comparing the regression model with and without video the statistic is $R = 9.89$ (p -value based on χ^2_1 of .002). We conclude the average number of ideas is different, with summary statistics suggesting that virtual pairs generate fewer ideas.

If we had resorted to a two sample t -test, we would have found a mean difference in the number of creative idea of $\Delta M = 1.19$, 95% CI [0.43, 1.95], $t(299) = 3.09$, $p = .002$.

Both tests come with slightly different sets of assumptions, but yield similar conclusions: there is evidence of a smaller number of creative ideas when people interact via videoconferencing.

Example 2.5 (Price of Spanish high speed train tickets). The Spanish national railway company, Renfe, manages regional and high speed train tickets all over Spain and The Gurus harvested the price of tickets sold by Renfe. We are interested in trips between Madrid and Barcelona and, for now, ask the question: are tickets more expensive one way or another? To answer this, we consider a sample of 8059 AVE tickets sold at Promo rate. Our test statistic will again be the mean difference between the price (in euros) for a train ticket for Madrid–Barcelona (μ_1) and the price for Barcelona–Madrid (μ_2), i.e., $\mu_1 - \mu_2$. The null hypothesis is that there are no difference in price, so $\mathcal{H}_0 : \mu_1 - \mu_2 = 0$.

We use Welch's t test statistic for two samples: the sample mean of the price of Barcelona–Madrid tickets is 82.15 euros, that of Madrid–Barcelona tickets is 82.54 euros and the Welch statistic is worth -1.15. If we use a normal approximation, the p -value is 0.25.

Rather than use the asymptotic distribution, whose validity stems from the central limit theorem, we could consider another approximation under the less restrictive assumption that the data are exchangeable: under the null hypothesis, there is no difference between the two destinations and so the label for destination (a binary indicator) is arbitrary. The

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reasoning underlying permutation tests is as follows: to create a benchmark, we will consider observations with the same number in each group, but permuting the labels. We then compute the test statistic on each of these datasets. If there are only a handful in each group (fewer than 10), we could list all possible permutations of the data, but otherwise we can repeat this procedure many times, say 9999, to get a good approximation. This gives an approximate distribution from which we can extract the p -value by computing the rank of our statistic relative to the others.

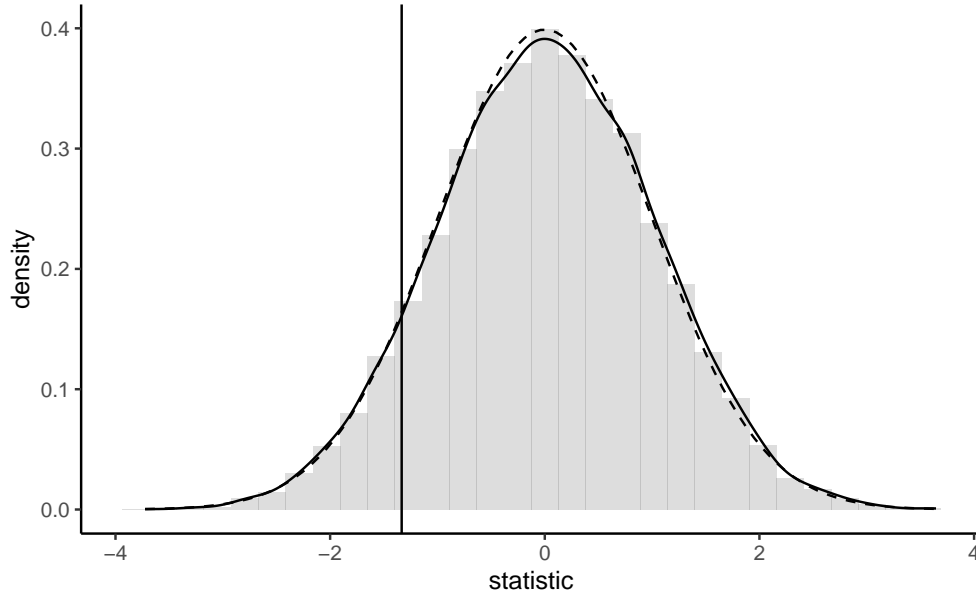


Figure 2.11: Permutation-based approximation to the null distribution of Welch two-sample t -test statistic (histogram and black curve) with standard normal approximation (dashed curve) for the price of AVE tickets at promotional rate between Madrid and Barcelona. The value of the test statistic calculated using the original sample is represented by a vertical line.

The so-called bootstrap approximation to the p -value of the permutation test, 0.186, is the proportion of statistics that are more extreme than the one based on the original sample. It is nearly identical to that obtained from the Satterthwaite approximation, 0.249 (the Student- t distribution is numerically equivalent to a standard normal with that many degrees of freedom), as shown in Figure 2.11. Even if our sample is very large ($n = 8059$ observations), the difference is not statistically significant. With a bigger sample (the database has more than 2 million tickets), we could estimate more precisely the average difference, up to 1/100 of an euro: the price difference would eventually become statistically significant, but this says nothing about practical difference: 0.28 euros relative to an

2.9 Examples

Promo ticket priced on average 82.56 euros is a negligible amount.

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