Chapter 3: Testing of Hypothesis

1 Basics of Hypothesis Testing

- A curious question: Does my data come from a prescribed distribution, F?
- This is often called testing goodness of fit.
- Example: You roll a 6-sided die n times and observe $1, 3, 1, 6, 4, 2, 5, 3, \ldots$ Is this a fair die?

Motivating Example: Einstein's theory of Brownian motion[1]

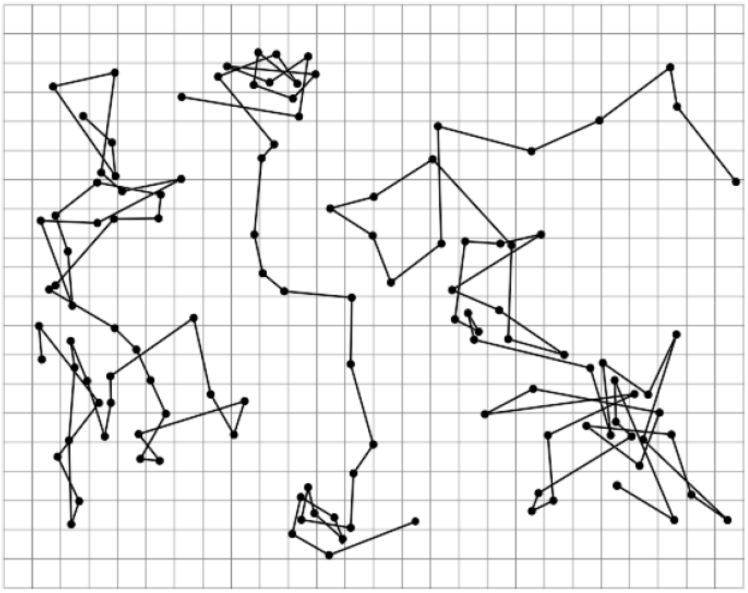


Figure 1: Motion of a tiny (radius $\approx 10^{-4}$ cm) particle suspended in water.

Albert Einstein (1905): $P_{t+\Delta t} \sim \mathcal{N}\left(P_t, \begin{pmatrix} \sigma^2 & 0 \\ 0 & \sigma^2 \end{pmatrix}\right)$, where $\sigma^2 = \frac{RT}{3\pi\eta r N_A}(\Delta t)$.

- P_t : position of particle at time t
- R: ideal gas constant
- T: absolute temperature
- η : viscosity of water
- r : radius of particle
- N_A : Avogadro's number

[1] Reading: A History of Random Processes (1968): https://www.jstor.org/stable/41133279

Jean Perrin (1909): Measured the position of a particle every 30 seconds to verify Einstein's theory (and to compute N_A). For his experiment, $\sigma^2 = 2.23 \times 10^{-7}$ cm^2.

- Does Perrin's data fit with Einstein's model?
- We will try to answer this question from testing of hypothesis viewpoint.

1.1 Null vs. Alternative Hypothesis

- A **hypothesis test** is a binary question about the data distribution. Our goal is to either accept a **null hypothesis** H_0 (which specifies something about this distribution) or to reject it in favor of an **alternative hypothesis** H_1.
- If H_0 (similarly H_1) completely specifies the probability distribution for the data, then the hypothesis is **simple**. Otherwise, it is **composite**.

1.2 Simple vs. Composite Hypothesis

Example 1.2.1. *Let $X_1, \ldots, X_6$ be the number of times we obtain 1 to 6 in n dice rolls. This null hypothesis is simple:*

$$H_0 : (X_1, \ldots, X_6) \sim \text{Multinomial}\left(n, \left(\frac{1}{6}, \ldots, \frac{1}{6}\right)\right).$$

We might wish to test this null hypothesis against the simple alternative hypothesis

$$H_1 : (X_1, \ldots, X_6) \sim \text{Multinomial}\left(n, \left(\frac{1}{9}, \frac{1}{9}, \frac{1}{9}, \frac{2}{9}, \frac{2}{9}, \frac{2}{9}\right)\right),$$

or perhaps against the composite alternative hypothesis

$$H_1 : (X_1, \ldots, X_6) \sim \text{Multinomial}\left(n, (p_1, \ldots, p_6)\right) \quad \textit{for some } (p_1, \ldots, p_6) \neq \left(\frac{1}{6}, \ldots, \frac{1}{6}\right).$$

Example 1.2.2. *Let $(X_1, Y_1), (X_2, Y_2), (X_3, Y_3), \ldots$ be the displacement vectors $P_{30} - P_0, P_{60} - P_{30}, P_{90} - P_{60}, \ldots$ where $P_t \in \mathbb{R}^2$ is the position of a particle at time t in Perrin's experiment. Einstein's theory corresponds to the simple null hypothesis*

$$H_0 : (X_1, Y_1), \ldots, (X_n, Y_n) \overset{IID}{\sim} \mathcal{N}\left(0, 2.23 \times 10^{-7} I\right).$$

To test the theory qualitatively, but possibly allow for an error in Einstein's formula for σ^2, we might test the composite null hypothesis

$$H_0 : (X_1, Y_1), \ldots, (X_n, Y_n) \overset{IID}{\sim} \mathcal{N}\left(0, \sigma^2 I\right) \textit{ for some } \sigma^2 > 0.$$

One can pose a number of different possible alternative hypotheses H_1 to the above nulls (to be discussed later).

1.3 Test statistics

A **test statistic** $T := T(X_1, \ldots, X_n)$ is any statistic such that extreme values (large or small) of T provide evidence against H_0.

Example 1.3.1. *Let $X_1, \ldots, X_6$ count the results from n dice rolls, and let*

$$T = \left(\frac{X_1}{n} - \frac{1}{6}\right)^2 + \ldots + \left(\frac{X_6}{n} - \frac{1}{6}\right)^2.$$

Large values of T provide evidence against the null hypothesis of a fair die,

$$H_0 : (X_1, \ldots, X_6) \sim \text{Multinomial}\left(n, \left(\frac{1}{6}, \ldots, \frac{1}{6}\right)\right).$$

Example 1.3.2. *Let $(X_1, Y_1), \ldots, (X_n, Y_n)$ be the displacements from Perrin's experiment*[2]*. To test*

$$H_0 : (X_1, Y_1), \ldots, (X_n, Y_n) \overset{IID}{\sim} \mathcal{N}\left(0, 2.23 \times 10^{-7} I\right)$$

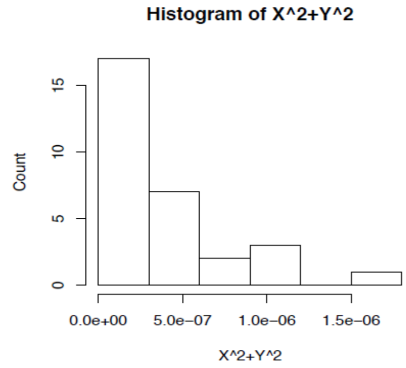
the following are possible test statistics:

$$\begin{aligned}
\bar{X} &= \frac{1}{n}(X_1 + \ldots + X_n) \\
\bar{Y} &= \frac{1}{n}(Y_1 + \ldots + Y_n) \\
V &= \frac{1}{n}\left(X_1^2 + Y_1^2 + \ldots + X_n^2 + Y_n^2\right)
\end{aligned}$$

(Values of $\bar{X}$ or $\bar{Y}$ much larger or smaller than 0 , or values of V much larger or smaller than $2 \times 2.23 \times 10^{-7}$, provide evidence against H_0 in favor of various alternatives H_1.)

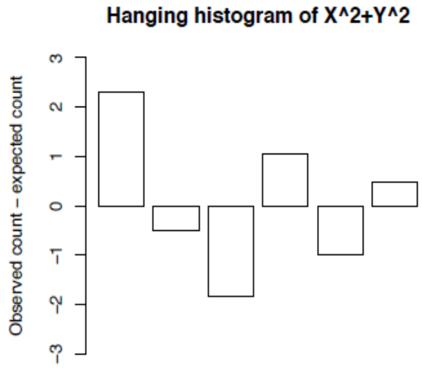
1.4 Test statistics from histograms

Let $R_i = X_i^2 + Y_i^2$. Suppose we are interested in testing whether $R_1, \ldots, R_n$ are distributed as $2.23 \times 10^{-7}\chi_2^2$ (their distribution under H_0). We can plot a histogram of these values:



Deviations from $2.23 \times 10^{-7}\chi_2^2$ are better visualized by a hanging histogram, which plots $O_i - E_i$ where O_i is the observed count for bin i and E_i is the expected count under the $2.23 \times 10^{-7}\chi_2^2$ distribution:

[2] Class Reading: https://aapt.scitation.org/doi/10.1119/1.2188962



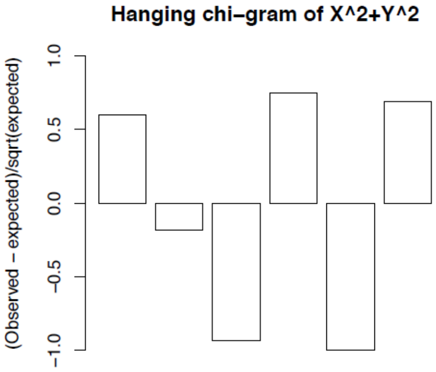
A test statistic can be $T=\sum_{i=1}^{6}\left(O_i-E_i\right)^2$.

Problem: Let p_i be the probability that the hypothesized chi-squared distribution assigns to bin i. If H_0 were true, then $O_i\sim\text{Binomial}\left(n,p_i\right)$ and $E_i=np_i=\mathbb{E}\left[O_i\right]$. So $\text{Var}\left[O_i\right]=\mathbb{E}\left[\left(O_i-E_i\right)^2\right]=np_i\left(1-p_i\right)$. The variation in O_i is smaller and scales approximately linearly with p_i if p_i is close to 0. This might explain why the bars were smaller on the right side of the hanging histogram.

Solution: We can "stabilize the variance" by looking at

$$\frac{O_i-E_i}{\sqrt{E_i}}=\frac{O_i-E_i}{\sqrt{np_i}}$$

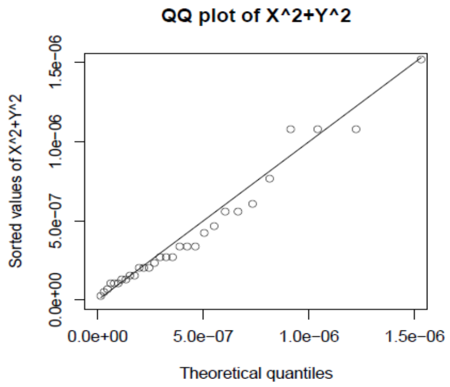
Or alternatively, we can look at $\sqrt{O_i}-\sqrt{E_i}$. (Taylor expansion of $\sqrt{x}$ around $x=E_i$ yields $\sqrt{O_i}-\sqrt{E_i}\approx\frac{1}{2\sqrt{E_i}}\left(O_i-E_i\right)$, so this has a similar effect as $\frac{O_i-E_i}{2\sqrt{E_i}}$ when O_i-E_i is small.) The hanging chi-gram plots $\frac{O_i-E_i}{\sqrt{E_i}}$:

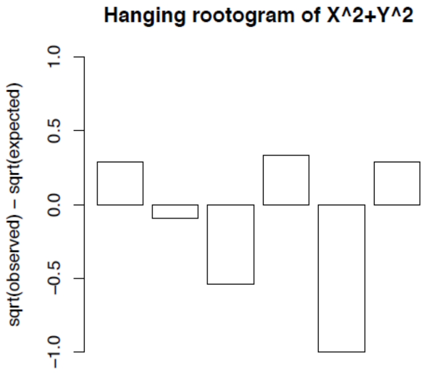


The test statistic $T=\sum_{i=1}^{6}\frac{\left(O_i-E_i\right)^2}{E_i}$ is called **Pearson's chi-squared statistic for goodness of fit.**[3]

Tukey's hanging rootogram plots $\sqrt{O_i}-\sqrt{E_i}$:

[3] Xu, M., Zhang, D., & Wu, W. B. (2019). Pearson's chi-squared statistics: approximation theory and beyond. Biometrika, 106(3), 716-723.





We may take as test statistic $T = \sum_{i=1}^{6}\left(\sqrt{O_i} - \sqrt{E_i}\right)^2$.

1.5 Test statistics from QQ plots

A **QQ plot** (or probability plot) compares the sorted values of $R_1, \ldots, R_n$ with the $\frac{1}{n+1}, \frac{2}{n+1}, \ldots, \frac{n}{n+1}$ quantiles of the hypothesized $2.23 \times 10^{-7}\chi_2^2$ distribution:
Values close to the line $y = x$ indicate a good fit.

How do we get a test statistic from a QQ plot? One way is to take the maximum vertical deviation from the $y = x$ line: Let $R_{(1)} < \ldots < R_{(n)}$ be the sorted values of $R_1, \ldots, R_n$. Take

$$T = \max_{i=1}^{n}\left|R_{(i)} - F^{-1}\left(\frac{i}{n+1}\right)\right|,$$

where F is the CDF of the $2.23 \times 10^{-7}\chi_2^2$ distribution so $F^{-1}(t)$ is its t^{th} quantile.

Problem: For values of R where the distribution has high density, the quantiles are closer together, so we expect a smaller vertical deviation. This explains why we see more vertical deviation in the upper right of the last QQ plot.

Solution: We may stabilize the spacings between quantiles by considering instead

$$T = \max_{i=1}^{n}\left|F\left(R_{(i)}\right) - \frac{i}{n+1}\right|.$$

This is almost the same as the **one-sample Kolmogorov-Smirnov (K-S) statistic**,

$$T_{KS} = \max_{i=1}^{n}\ \max\left(\left|F\left(R_{(i)}\right) - \frac{i}{n}\right|, \left|F\left(R_{(i)}\right) - \frac{i-1}{n}\right|\right).$$

(You can show $\frac{i-1}{n} < \frac{i}{n+1} < \frac{i}{n}$, and the difference between T and T_{KS} is negligible for large n.)

1.6 Null distributions and type I error

Supposing that we've picked our test statistic T, how large (or small) does T need to be, before we can safely assert that H_0 is false?

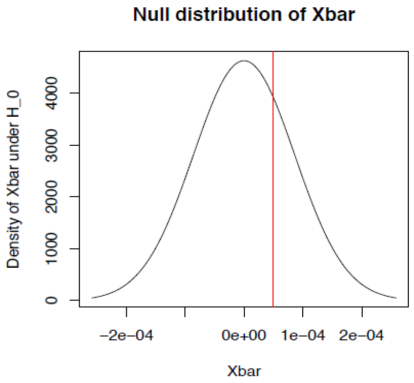
In most cases, we can never be 100% sure that H_0 is false. But we can compute T from the observed data and compare it with the sampling distribution of T if H_0 were true. This is called the **null distribution** of T.

Example 1.6.1. *Consider the following null hypothesis*

$$H_0 : (X_1, Y_1), \ldots, (X_n, Y_n) \overset{IID}{\sim} \mathcal{N}\left(0, 2.23 \times 10^{-7} I\right).$$

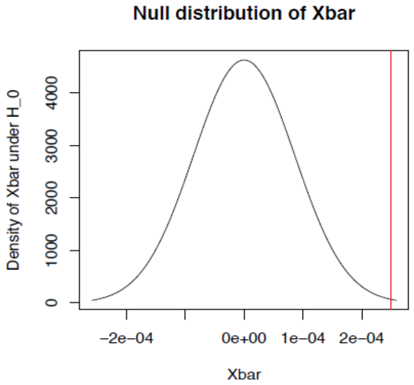
Under $H_0, \bar{X} \sim \mathcal{N}(0, 2.23 \times 10^{-7}/n)$. *This normal distribution is the null distribution of* $\bar{X}$.

Here's the PDF for the null distribution of $\bar{X}$, when $n = 30$:



If, for the observed data, $\bar{X} = 0.5 \times 10^{-4}$, this would not provide strong evidence against H_0. In this case, we might accept H_0.

Here's the PDF for the null distribution of $\bar{X}$, when $n = 30$:



If, for the observed data, $\bar{X} = 2.5 \times 10^{-4}$, this would provide strong evidence against H_0. In this case we might reject H_0.

The **rejection region** is the set of values of T for which we choose to reject H_0. The **acceptance region** is the set of values of T for which we choose to accept H_0.

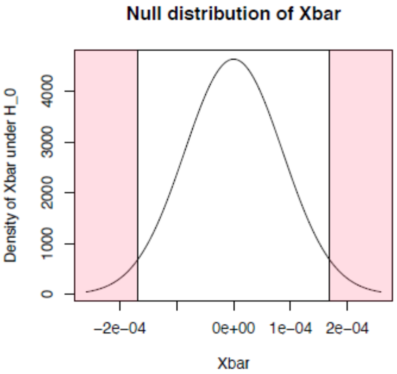
We choose the rejection region so as to control the probability of **type I error**:

$$\alpha = \mathbb{P}_{H_0}\left[\text{reject } H_0\right].$$

This value α is also called the **significance level** of the test.

If, under its null distribution, T belongs to the rejection region with probability α, then the test is level-α.

(Notation: For a simple null hypothesis H_0, we write $\mathbb{P}_{H_0}[\mathcal{E}]$ to denote the probability of event $\mathcal{E}$ under H_0, i.e., the probability of $\mathcal{E}$ if H_0 were true.)



Example 1.6.2. *A (two-sided) level-α test might reject H_0 when $\bar{X}$ falls in the above shaded regions. Mathematically, let z_α denote the $1-\alpha$ quantile, or "upper α point", of the distribution $\mathcal{N}(0,1)$. As $\bar{X} \sim \mathcal{N}\left(0, \sigma^2/n\right)$ under H_0 (where $\sigma^2 = 2.23 \times 10^{-7}$), the rejection region should be $\left(-\infty, -\frac{\sigma}{\sqrt{n}} \times z_{\alpha/2}\right] \cup \left[\frac{\sigma}{\sqrt{n}} \times z_{\alpha/2}, \infty\right)$.*

1.7 P values

The **p-value** is the smallest significance level at which your test would have rejected H_0.

For a one-sided test that rejects for large T, letting t_{obs} denote the value of T computed from the observed data, the p-value is $\mathbb{P}_{H_0}\left[T \geq t_{\text{obs}}\right]$.

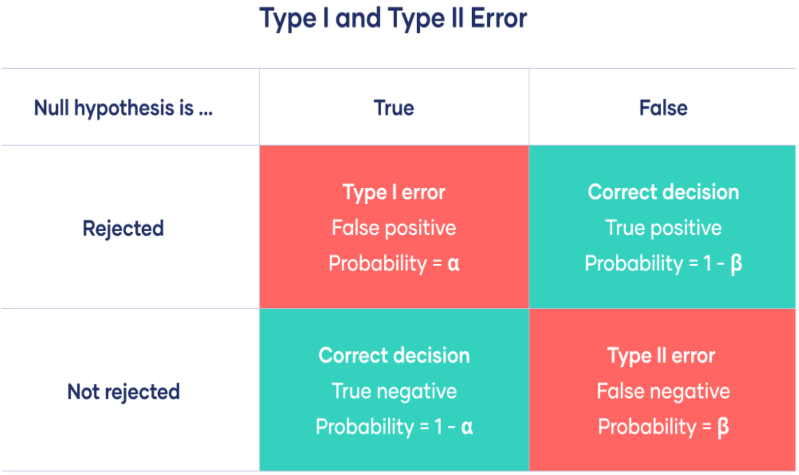
For a two-sided test that rejects at the $\alpha/2$ and $1-\alpha/2$ quantiles of the null distribution of T, the p-value is 2 times the smaller of $\mathbb{P}_{H_0}\left[T \geq t_{\text{obs}}\right]$ and $\mathbb{P}_{H_0}\left[T \leq t_{\text{obs}}\right]$.

The p-value provides a quantitative measure of the extent to which the data supports (or does not support) H_0. It is preferable to report the exact p-value, rather than to just say "we rejected at level-0.05".

1.8 A word of caution

Accepting (or failing to reject) H_0 **does not** imply there is strong evidence that H_0 is true. Both of the following are possible:

- The particular test statistic you chose is not good at distinguishing the null hypothesis H_0 from the true distribution. Or equivalently, the true distribution is not well-captured by the alternative H_1 that your test statistic is targeting. (For example, in **Perrin's data**, if there is significant drift in the y direction, you would not detect this using the test statistic $\bar{X}$.)
- You do not have enough data to reject H_0 at the significance level that you desire. In this case, your study might be **underpowered** (to be discussed later).



Type I and Type II Error

Null hypothesis is ...	True	False
Rejected	Type I error False positive Probability = α	Correct decision True positive Probability = 1 - β
Not rejected	Correct decision True negative Probability = 1 - α	Type II error False negative Probability = β

1.9 Determining the null distribution

To figure out the rejection region, we must understand the null distribution of the test statistic. There are three methods:

- Sometimes we can derive the null distribution exactly, for some of the previous problem where the test statistic is $\bar{X}$ and $X_1, \ldots, X_n$ are normally distributed under H_0.
- Sometimes we can derive an asymptotic approximation, using tools such as the CLT and continuous mapping theorem.
- When H_0 is simple, we can always obtain the null distribution by simulation.

1.10 Using an asymptotic null distribution

Example 1.10.1. *Let $(X_1, \ldots, X_6)$ denote the counts of 1 to 6 from n rolls of a die, and consider testing the simple null of a fair die*

$$H_0 : (X_1, \ldots, X_6) \sim \text{Multinomial}\left(n, \left(\frac{1}{6}, \ldots, \frac{1}{6}\right)\right)$$

using the test statistic

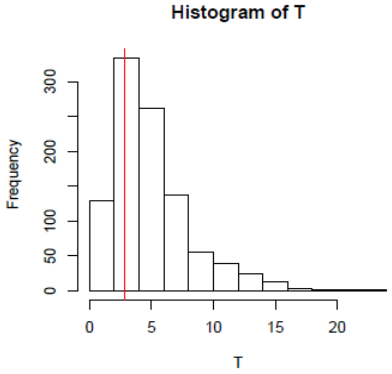
$$T = \left(\frac{X_1}{n} - \frac{1}{6}\right)^2 + \ldots + \left(\frac{X_6}{n} - \frac{1}{6}\right)^2.$$

It can be shown that for large n, T is approximately distributed as $\frac{1}{6n}\chi_5^2$.

*To perform an **asymptotic level-α test**, we may reject H_0 when t_{obs} exceeds $\frac{1}{6n}\chi_5^2(\alpha)$, where $\chi_n^2(\alpha)$ denotes the $1 - \alpha$ quantile, or "upper α point", of the χ_n^2 distribution.*

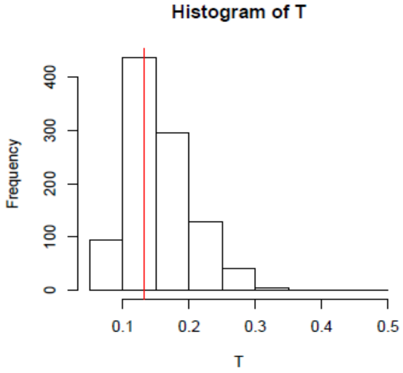
1.11 Using a simulated null distribution

Example 1.11.1. *Let T be Pearson's chi-squared statistic for goodness of fit for the values $X_1^2 + Y_1^2, \ldots, X_{30}^2 + Y_{30}^2$ from Perrin's experiments. We may simulate the null distribution of T :*



This shows the 1000 values of T across 1000 simulations. The observed value $t_{\text{obs}} = 2.83$ for Perrin's real data is in red.

Example 1.11.2. *Let T be the K-S statistic for $X_1^2 + Y_1^2, \ldots, X_{30}^2 + Y_{30}^2$. We may simulate the null distribution of T :*



The observed value $t_{\text{obs}} = 0.132$ for Perrin's real data is in red.

We obtain an approximate p-value as the fraction of simulated values of T larger than t_{obs}. (For a two-sided test, we would take either the fraction of simulated values of T larger than t_{obs} or smaller than t_{obs}, and multiply this by 2.)

For Perrin's data, the Pearson chi-squared p-value is 0.754, and the K-S p-value is 0.612. We accept H_0 in both cases, and neither test provides significant evidence against Einstein's theory of Brownian motion.

2 Simple alternatives and the Neyman-Pearson lemma

- Till now, we discussed a number of ways to construct test statistics for testing a simple null hypothesis, and we showed how to use the null distribution of the statistic to determine the rejection region so as to achieve the desired significance level.

- In this section, our goal is to answer the following question: Which test statistic should we use?
- The answer depends on the alternative hypothesis that we wish to distinguish from the null.

2.1 The Neyman-Pearson lemma

Let's focus on the problem of testing a simple null hypothesis H_0 against a simple alternative hypothesis H_1. We denote by

$$\beta = \mathbb{P}_{H_1}\left[\text{accept } H_0\right],$$

the probability of **type II error** – accepting the null H_0 when in fact the alternative H_1 is true. (Here, $\mathbb{P}_{H_1}[\mathcal{E}]$ denotes probability of an event $\mathcal{E}$ if H_1 is true.) Equivalently,

$$1 - \beta = \mathbb{P}_{H_1}\left[\text{reject } H_0\right],$$

is the probability of correctly rejecting H_0 when H_1 is true, which is called the **power** of the test against H_1.[4] When designing a hypothesis test for testing H_0 versus H_1, we have the following goal:

$$\begin{aligned}&\text{maximize: the power of the test against } H_1\\ &\text{subject to: the significance level of the test under } H_0 \text{ is at most } \alpha.\end{aligned}$$

This is an example of a constrained optimization problem, which we can reason about in the following way: Suppose we observe data which are realizations of random variables $X_1, \ldots, X_n$. For notational convenience, let us denote by $\mathbf{X} = (X_1, \ldots, X_n)$ the entire data vector, and by $\mathbf{x} = (x_1, \ldots, x_n)$ a vector of possible values for $\mathbf{X}$. In the discrete case, suppose the hypotheses are

$$\begin{aligned}H_0 &: \mathbf{X} \text{ is distributed with joint PMF } f_0(\mathbf{x}) := f_0\left(x_1, \ldots, x_n\right),\\ H_1 &: \mathbf{X} \text{ is distributed with joint PMF } f_1(\mathbf{x}) := f_1\left(x_1, \ldots, x_n\right).\end{aligned}$$

Let $\mathcal{X}$ denote the set of all possible values of $\mathbf{X}$ under f_0 and f_1. To define the hypothesis test, for each $\mathbf{x} \in \mathcal{X}$, we must specify whether to accept or reject H_0 if the observed data is $\mathbf{x}$. In other words, we specify a rejection region $\mathcal{R} \subset \mathcal{X}$ such that we reject H_0 if the observed data belongs to $\mathcal{R}$ and we accept H_0 otherwise. Then the probability of rejecting H_0 if H_0 were true would be $\sum_{\mathbf{x}\in\mathcal{R}} f_0(\mathbf{x})$, the probability of rejecting H_0 if H_1 were true would be $\sum_{\mathbf{x}\in\mathcal{R}} f_1(\mathbf{x})$, and the above optimization problem is formalized as choosing the rejection region $\mathcal{R} \subset \mathcal{X}$ with the goal

$$\begin{aligned}\text{maximize} \sum_{\mathbf{x}\in\mathcal{R}} f_1(\mathbf{x})\\ \text{subject to} \sum_{\mathbf{x}\in\mathcal{R}} f_0(\mathbf{x}) \le \alpha.\end{aligned}$$

The continuous case is similar: suppose the hypotheses are

$$\begin{aligned}H_0 &: \mathbf{X} \text{ is distributed with joint PDF } f_0(\mathbf{x}) := f_0\left(x_1, \ldots, x_n\right),\\ H_1 &: \mathbf{X} \text{ is distributed with joint PDF } f_1(\mathbf{x}) := f_1\left(x_1, \ldots, x_n\right).\end{aligned}$$

We define a hypothesis test by defining the region $\mathcal{R} \subset \mathbb{R}^n$ such that we reject H_0 if and only if the observed data $\mathbf{x}$ belongs to $\mathcal{R}$. The above optimization problem is to choose $\mathcal{R} \subset \mathbb{R}^n$ with the goal

[4] Caution: Some books/papers use opposite notation and let β denote the power and $1-\beta$ denote the probability of type II error. Make sure to double-check the meaning of the notation.

$$\text{maximize} \int_{\mathcal{R}} f_1(\mathbf{x}) dx_1 \dots dx_n$$
$$\text{subject to} \int_{\mathcal{R}} f_0(\mathbf{x}) dx_1 \dots dx_n \leq \alpha.$$

In either the discrete or continuous case, what are the best points $\mathbf{x}$ to include in this rejection region $\mathcal{R}$? A moment's thought should convince you that $\mathcal{R}$ should consist of those points $\mathbf{x}$ corresponding to the smallest values of $\frac{f_0(\mathbf{x})}{f_1(\mathbf{x})}$, as these give the "smallest increase in type I error per unit increase of power". Another interpretation is that these are the points providing the strongest evidence in favor of H_1 over H_0. The statistic

$$L(\mathbf{X}) = \frac{f_0(\mathbf{X})}{f_1(\mathbf{X})}$$

is called the ***likelihood ratio statistic***, and the test that rejects for small values of $L(\mathbf{X})$ is called the ***likelihood ratio test***. The Neyman-Pearson lemma shows that the likelihood ratio test is the most powerful test of H_0 against H_1:

Theorem 2.1. *(Neyman-Pearson lemma). Let H_0 and H_1 be simple hypotheses (in which the data distributions are either both discrete or both continuous). For a constant $c > 0$, suppose that the likelihood ratio test which rejects H_0 when $L(\mathbf{x}) < c$ has significance level α. Then for any other test of H_0 with significance level at most α, its power against H_1 is at most the power of this likelihood ratio test.*

Proof. Consider the discrete case, and let $\mathcal{R} = \{\mathbf{x} : L(\mathbf{x}) < c\}$ be the rejection region of the likelihood ratio test. Note that among all subsets of $\mathcal{X}, \mathcal{R}$ maximizes the quantity

$$\sum_{x \in \mathcal{R}} (cf_1(\mathbf{x}) - f_0(\mathbf{x})),$$

because $cf_1(\mathbf{x}) - f_0(\mathbf{x}) > 0$ for $\mathbf{x} \in \mathcal{R}$ and $cf_1(\mathbf{x}) - f_0(\mathbf{x}) \leq 0$ for $\mathbf{x} \notin \mathcal{R}$. Hence for any other test with significance level at most α, say with rejection region $\mathcal{R}'$,

$$\sum_{x \in \mathcal{R}} (cf_1(\mathbf{x}) - f_0(\mathbf{x})) \geq \sum_{x \in \mathcal{R}'} (cf_1(\mathbf{x}) - f_0(\mathbf{x})).$$

Rearranging the above, this implies

$$c \left(\sum_{x \in \mathcal{R}} f_1(\mathbf{x}) - \sum_{x \in \mathcal{R}'} f_1(\mathbf{x}) \right) \geq \sum_{x \in \mathcal{R}} f_0(\mathbf{x}) - \sum_{x \in \mathcal{R}'} f_0(\mathbf{x}) = \alpha - \sum_{x \in \mathcal{R}'} f_0(\mathbf{x}) \geq 0,$$

where the last inequality follows because $\sum_{x \in \mathcal{R}'} f_0(\mathbf{x})$ is the significance level of the test that rejects for $\mathbf{x} \in \mathcal{R}'$. Then $\sum_{x \in \mathcal{R}} f_1(\mathbf{x}) \geq \sum_{x \in \mathcal{R}'} f_1(\mathbf{x})$, i.e. the likelihood ratio test has power at least that of this other test. The proof in the continuous case is exactly the same, with all sums above replaced by integrals over $\mathcal{R}$ and $\mathcal{R}'$. □

2.2 Examples

Let's work out what the likelihood ratio test actually is for two simple examples.

Example 2.2.1. *Consider data $X_1, \ldots, X_n$ and the following null and alternative hypotheses:*

$$H_0 : X_1, \ldots, X_n \overset{IID}{\sim} \mathcal{N}(0,1)$$
$$H_1 : X_1, \ldots, X_n \overset{IID}{\sim} \mathcal{N}(\mu,1).$$

Here we assume μ is a known, specified value (not equal to 0), so that H_1 is a simple alternative hypothesis. The joint PDF *of $(X_1, \ldots, X_n)$ under H_0 is*

$$f_0\left(x_1, \ldots, x_n\right) = \prod_{i=1}^{n} \left(\frac{1}{\sqrt{2\pi}} e^{-\frac{x_i^2}{2}} \right) = \left(\frac{1}{\sqrt{2\pi}} \right)^n \exp\left(-\frac{x_1^2 + \ldots + x_n^2}{2} \right).$$

The joint PDF under H_1 is

$$f_1\left(x_1, \ldots, x_n\right) = \prod_{i=1}^{n} \left(\frac{1}{\sqrt{2\pi}} e^{-\frac{(x_i-\mu)^2}{2}} \right) = \left(\frac{1}{\sqrt{2\pi}} \right)^n \exp\left(-\frac{\left(x_1-\mu\right)^2 + \ldots + \left(x_n-\mu\right)^2}{2} \right).$$

Thus, the likelihood ratio statistic is

$$L\left(X_1, \ldots, X_n\right) = \frac{f_0\left(X_1, \ldots, X_n\right)}{f_1\left(X_1, \ldots, X_n\right)} = \exp\left(-\frac{X_1^2 + \ldots + X_n^2}{2} + \frac{\left(X_1-\mu\right)^2 + \ldots + \left(X_n-\mu\right)^2}{2} \right).$$

By expanding the squares and simplifying, we obtain

$$L\left(X_1, \ldots, X_n\right) = \exp\left(\frac{-2\mu\left(X_1 + \ldots + X_n\right) + n\mu^2}{2} \right).$$

Suppose first that $\mu > 0$. Then $L\left(X_1, \ldots, X_n\right)$ is a strictly decreasing function of the sample mean $\bar{X} = \frac{1}{n}\left(X_1 + \ldots + X_n\right)$. Hence, rejecting for small values of $L\left(X_1, \ldots, X_n\right)$ is the same as rejecting for large values of $\bar{X}$. So the Neyman-Pearson lemma tells us that the most powerful test should reject when $\bar{X} > c$, for some threshold c. We pick c to ensure that the significance level is α under H_0: Since the null distribution of $\bar{X}$ is $\bar{X} \sim \mathcal{N}\left(0, \frac{1}{n}\right)$, c should be the $\frac{1}{\sqrt{n}} z_\alpha$ where z_α is the "upper α point" of the standard normal distribution.

Now suppose that $\mu < 0$. Then $L\left(X_1, \ldots, X_n\right)$ is strictly increasing in $\bar{X}$, so rejecting for small $L\left(X_1, \ldots, X_n\right)$ is the same as rejecting for small $\bar{X}$. By the same argument as above, to ensure significance level α, the most powerful test rejects when $\bar{X} < -\frac{1}{\sqrt{n}} z_\alpha$.

Remark 2.1. *The most powerful test against the alternative $H_1 : X_1, \ldots, X_n \sim \mathcal{N}(\mu, 1)$ is the same for any $\mu > 0$ (rejecting when $\bar{X} > \frac{1}{\sqrt{n}} z_\alpha$), and neither the test statistic nor the rejection region depends on the specific value of μ. This means that, in fact, this test is uniformly most powerful (UMP) against the (one-sided) composite alternative*

$$H_1 : X_1, \ldots, X_n \overset{IID}{\sim} \mathcal{N}(\mu, 1) \text{ for some } \mu > 0.$$

On the other hand, the most powerful test is different for $\mu > 0$ versus for $\mu < 0$: one test rejects for large positive values of $\bar{X}$, and the other rejects for large negative values of $\bar{X}$. This implies that there does not exist a single most powerful test for the (two-sided) composite alternative

$$H_1 : X_1, \ldots, X_n \overset{IID}{\sim} \mathcal{N}(\mu, 1) \text{ for some } \mu \neq 0.$$

Example 2.2.2. *Let $X_1, \ldots, X_n \in \{0,1\}$ be the results of n flips of a coin, and consider the following null and alternative hypotheses:*

$$H_0 : X_1, \ldots, X_n \overset{IID}{\sim} \text{Bernoulli}\left(\frac{1}{2}\right)$$
$$H_1 : X_1, \ldots, X_n \overset{IID}{\sim} \text{Bernoulli}(p).$$

Here we assume that $p \neq \frac{1}{2}$ is a known and specified value, so H_1 is simple. The joint PMF of $(X_1, \ldots, X_n)$ under H_0 and H_1 are, respectively,

$$f_0\left(x_1, \ldots, x_n\right) = \prod_{i=1}^{n} \frac{1}{2} = \frac{1}{2^n},$$
$$f_1\left(x_1, \ldots, x_n\right) = \prod_{i=1}^{n} p^{x_i}(1-p)^{1-x_i} = p^{x_1+\ldots+x_n}(1-p)^{n-x_1-\ldots-x_n} = (1-p)^n\left(\frac{p}{1-p}\right)^{x_1+\ldots+x_n}.$$

Thus, the likelihood ratio statistic is

$$L\left(X_1, \ldots, X_n\right) = \frac{f_0\left(X_1, \ldots, X_n\right)}{f_1\left(X_1, \ldots, X_n\right)} = \frac{1}{2^n(1-p)^n}\left(\frac{1-p}{p}\right)^{X_1+\ldots+X_n}.$$

First suppose $p > \frac{1}{2}$. Then $L\left(X_1, \ldots, X_n\right)$ is a decreasing function of $S = X_1 + \ldots + X_n$, so rejecting for small values of $L\left(X_1, \ldots, X_n\right)$ is the same as rejecting for large values of S. Hence, by the Neyman-Pearson lemma, the most powerful test rejects when $S > c$ for a constant c. We choose c to ensure significance level α : Under H_0, *$S \sim$* Binomial $\left(n, \frac{1}{2}\right)$*, so c should be the $1-\alpha$ quantile of the* Binomial $\left(n, \frac{1}{2}\right)$ *distribution. This test is the same for all $p > \frac{1}{2}$, so it is in fact uniformly most powerful against the composite alternative*

$$H_1 : X_1, \ldots, X_n \overset{IID}{\sim} \text{Bernoulli}(p) \textit{ for some } p > \frac{1}{2}.$$

For $p < \frac{1}{2}$, $L\left(X_1, \ldots, X_n\right)$ is increasing in S, so the most powerful test rejects for $S < c$ and some constant c. To ensure significance level α, c should be the α quantile of the Binomial $\left(n, \frac{1}{2}\right)$ *distribution. This test is the same for all $p < \frac{1}{2}$, so it is uniformly most powerful against the compositive alternative*

$$H_1 : X_1, \ldots, X_n \overset{IID}{\sim} \text{Bernoulli}(p) \textit{ for some } p < \frac{1}{2}.$$

Remark 2.2. *We have glossed over a detail, which is that when the distribution of the likelihood ratio statistic $L(\mathbf{X})$ is discrete under H_0, it might not be possible to choose c so that the significance level is exactly α. For instance, in the previous example, suppose we wish to achieve significance level $\alpha = 0.05$, and $n = 20$. For $S \sim$* Binomial $\left(20, \frac{1}{2}\right)$*, we have $\mathbb{P}[S \geq 15] = 0.021$ and $\mathbb{P}[S \geq 14] = 0.058$. So if we reject H_0 when $S \geq 14$, we do not achieve significance level $\leq \alpha$, and if we reject H_0 when $S \geq 15$, then we are too conservative.*

The theoretically correct solution is to perform a randomized test: Always reject H_0 when $S \geq 15$, always accept H_0 when $S \leq 13$, and reject H_0 with a certain probability when $S = 14$, where this probability is chosen to make the significance level exactly α. A more complete statement of the Neyman-Pearson lemma shows that this type of (possibly randomized) likelihood ratio test is most powerful among all randomized tests.

In practice, it might not be acceptable to use a randomized test. (We found the effects of this drug to be statistically significant because our statistical procedure told us to flip a coin, and our coin landed heads...) So we might take the more conservative option of just rejecting H_0 when $S \geq 15$.

3 Composite Hypotheses and the t-test

In this section we will discuss various hypothesis testing problems involving a composite null hypothesis and a composite alternative hypothesis.

3.1 Composite null and alternative hypotheses

To motivate the discussion, consider the following examples:

Example 3.1.1. *Suppose there are 80 students in a class taking MATH350 course. A diagnostic exam is administered at the start of the quarter, and a comparable exam is administered at the end of the quarter. Did the course MATH350 improve students' knowledge of statistics?*

Let X_i be the difference in test scores for student i. There are various ways we can formulate the above question as a hypothesis test: If we believe a normal model for the X_i's, $X_1, \ldots, X_{80} \overset{IID}{\sim} \mathcal{N}(\mu, \sigma^2)$, then we might formulate our question as the testing problem:

$$\begin{aligned} H_0 &: \mu = 0 \\ H_1 &: \mu > 0. \end{aligned}$$

Note that both the null and alternative hypotheses above are composite, because they do not specify the variance σ^2 (which is unknown). If we are not willing to make a normality assumption, we might assume instead that $X_1, \ldots, X_{80}$ are IID with PDF f, and test

$$\begin{aligned} H_0 &: f \text{ is symmetric around } 0 \\ H_1 &: f \text{ is symmetric around } \mu \text{ for some } \mu > 0 \end{aligned}$$

or maybe even drop the symmetry assumptions and test

$$\begin{aligned} H_0 &: f \text{ has median } 0 \\ H_1 &: f \text{ has median } \mu \text{ for some } \mu > 0. \end{aligned}$$

Which formulation we choose and the resulting test statistic we use may depend on our prior knowledge of how test scores are typically distributed and on visual inspection of the data. (for departures from normality, symmetry, etc.)

Example 3.1.2. *A friend criticizes the setup of the previous example: It's hard to make two exams that are equally difficult. What if the second exam just happened to be a bit easier?*

To address this criticism, we add a control group: We give 100 other students (who are not taking statistics courses this quarter) the same two exams at the start and end of the quarter. Let Y_i be the difference in test scores for student i of this control group. Again, if we believe a normal model $X_1, \ldots, X_{80} \overset{IID}{\sim} \mathcal{N}(\mu_X, \sigma^2)$ and $Y_1, \ldots, Y_{100} \overset{IID}{\sim} \mathcal{N}(\mu_Y, \sigma^2)$ (with the X's also independent of the Y's), then we might formulate the test as

$$\begin{aligned} H_0 &: \mu_X = \mu_Y \\ H_1 &: \mu_X > \mu_Y. \end{aligned}$$

If we are not willing to assume normality, we might suppose instead that $X_1, \ldots, X_{80}$ are IID with PDF f and $Y_1, \ldots, Y_{100}$ are IID with PDF g, and test

$$\begin{aligned} &H_0 : f = g \\ &H_1 : f \text{ stochastically dominates } g. \end{aligned}$$

(This alternative H_1 means that if $X \sim f$ and $Y \sim g$, then $\mathbb{P}[X \geq x] \geq \mathbb{P}[Y \geq x]$ for all $x \in \mathbb{R}$.) Again, how we formulate the testing problem depends on the modeling assumptions we are willing to make.

When testing a composite null hypothesis H_0 against a compositive alternative H_1, there is a probability of type I error associated to each data distribution $P \in H_0$ (the probability of rejecting H_0 if the true distribution were P) and a probability of type II error associated to each data distribution $P \in H_1$ (the probability of accepting H_0 if the true distribution were P). A test has **significance level** α if the maximum probability of type I error for any $P \in H_0$ is α.

This means that to design a level-α test of H_0, we need to control the probability of type I error for every $P \in H_0$, and hence reason about the sampling distribution of our test statistic T under every such data distribution P. In general, this can be very difficult, and a common simplifying strategy will be to find a test statistic T that has *the same* sampling distribution under every $P \in H_0$.

3.2 One-sample t-test

Assume $X_1, \ldots, X_n \overset{\text{IID}}{\sim} \mathcal{N}(\mu, \sigma^2)$ for unknown μ and σ^2, and consider testing

$$\begin{aligned} &H_0 : \mu = 0 \\ &H_1 : \mu > 0. \end{aligned}$$

If σ^2 were fixed and known, then the uniformly most-powerful level-α test would reject for large values of $\bar{X}$. Specifically, it would reject when $\frac{\sqrt{n}\bar{X}}{\sigma} > z_\alpha$ (because when
$X_1, \ldots, X_n \overset{IID}{\sim} \mathcal{N}(0, \sigma^2), \bar{X} \sim \mathcal{N}\left(0, \frac{\sigma^2}{n}\right)$ so $\frac{\sqrt{n}\bar{X}}{\sigma} \sim \mathcal{N}(0,1)$).
When σ^2 is unknown, a natural idea is to estimate σ^2 by the sample variance

$$S^2 = \frac{1}{n-1}\left(\left(X_1 - \bar{X}\right)^2 + \ldots + \left(X_n - \bar{X}\right)^2\right),$$

and to consider the test statistic

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

To derive the distribution of T under H_0, we first prove the following result:

Theorem 3.1. *Let $X_1, \ldots, X_n \overset{IID}{\sim} \mathcal{N}(\mu, \sigma^2)$, and let $\bar{X}$ and S^2 be the sample mean and sample variance (where S^2 is defined as above). Then S^2 is independent of $\bar{X}$ and distributed as $S^2 \sim \frac{\sigma^2}{n-1}\chi^2_{n-1}$.*

Proof. Note that changing the mean μ does not affect the distribution of S^2 and shifts the distribution of $\bar{X}$ by a constant value, which does not affect the independence of S^2 and $\bar{X}$. So, we may assume without loss of generality $\mu = 0$.

We first show independence of S^2 and $\bar{X}$. The entries of $\left(\bar{X}, X_1-\bar{X}, \ldots, X_n-\bar{X}\right)$ are linear combinations of $X_1, \ldots, X_n$, so $\left(\bar{X}, X_1-\bar{X}, \ldots, X_n-\bar{X}\right)$ has a multivariate normal distribution by Example 1.9 of Chapter 1. Let's compute

$$\operatorname{Cov}\left[\bar{X}, X_1-\bar{X}\right]=\operatorname{Cov}\left[\bar{X}, X_1\right]-\operatorname{Cov}[\bar{X}, \bar{X}].$$

By bilinearity of covariance and the fact that $\operatorname{Cov}\left[X_j, X_1\right]=0$ for all $j \geq 2$,

$$\begin{aligned}
\operatorname{Cov}\left[\bar{X}, X_1\right] &= \operatorname{Cov}\left[\frac{1}{n} \sum_{j=1}^n X_j, X_1\right] \\
&= \frac{1}{n} \sum_{j=1}^n \operatorname{Cov}\left[X_j, X_1\right]=\frac{1}{n} \operatorname{Cov}\left[X_1, X_1\right]=\frac{1}{n} \operatorname{Var}\left[X_1\right]=\frac{\sigma^2}{n}.
\end{aligned}$$

Since $\bar{X} \sim \mathcal{N}\left(0, \frac{\sigma^2}{n}\right)$, $\operatorname{Cov}[\bar{X}, \bar{X}]=\operatorname{Var}[\bar{X}]=\frac{\sigma^2}{n}$ also. Then

$$\operatorname{Cov}\left[\bar{X}, X_1-\bar{X}\right]=0.$$

Similarly $\operatorname{Cov}\left[\bar{X}, X_i-\bar{X}\right]=0$ for every $i=2, \ldots, n$. By Theorem 1.2 from Chapter 1, this means $\bar{X}$ is independent of $\left(X_1-\bar{X}, \ldots, X_n-\bar{X}\right)$, and so $\bar{X}$ is independent of S^2.

To compute the distribution of S^2, we may write

$$\begin{aligned}
(n-1) S^2 &= \left(X_1-\bar{X}\right)^2+\ldots+\left(X_n-\bar{X}\right)^2 \\
&= \left(X_1^2-2 X_1 \bar{X}+\bar{X}^2\right)+\ldots+\left(X_n^2-2 X_n \bar{X}+\bar{X}^2\right) \\
&= X_1^2+\ldots+X_n^2-2\left(X_1+\ldots+X_n\right) \bar{X}+n \bar{X}^2 \\
&= \left(X_1^2+\ldots+X_n^2\right)-2 n \bar{X}^2+n \bar{X}^2 \\
&= \left(X_1^2+\ldots+X_n^2\right)-n \bar{X}^2 .
\end{aligned}$$

Letting $U=(n-1) S^2 / \sigma^2$, $W=\left(X_1^2+\ldots+X_n^2\right) / \sigma^2$, and $V=n \bar{X}^2 / \sigma^2$, this says $W=U+V$. We showed S^2 is independent of $\bar{X}$, hence U is independent of V. Thus the MGF of W is the product of the MGFs of U and V:

$$M_W(t)=M_U(t) M_V(t).$$

Finally, note that each $X_i / \sigma \sim \mathcal{N}(0,1)$, so $W \sim \chi_n^2$. Also, $\sqrt{n} \bar{X} / \sigma \sim \mathcal{N}(0,1)$, so $V=(\sqrt{n} \bar{X} / \sigma)^2 \sim \chi_1^2$. This means that the MGF of U is, for any $t<\frac{1}{2}$,

$$M_U(t)=\frac{M_W(t)}{M_V(t)}=\frac{(1-2 t)^{-n / 2}}{(1-2 t)^{-1 / 2}}=(1-2 t)^{-(n-1) / 2},$$

which is the MGF of the χ_{n-1}^2 distribution. So $U \sim \chi_{n-1}^2$, and $S^2 \sim \frac{\sigma^2}{n-1} \chi_{n-1}^2$. □

Remark 3.1. *Previously, we claimed if $W_1, \ldots, W_6 \overset{IID}{\sim} \mathcal{N}(0,1)$, then $(W_1-\bar{W})^2+\ldots+\left(W_6-\bar{W}\right)^2 \sim \chi_5^2$. The above theorem verifies this. The theorem also explains why we often define S^2 with the normalization $\frac{1}{n-1}$ rather than $\frac{1}{n}$: As the expectation of a χ_{n-1}^2 random variable is $n-1, \mathbb{E}\left[S^2\right]=\sigma^2$ so S^2 is an unbiased estimator for σ^2. Returning to our test statistic*

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

we observe that by Theorem 3.1, for $\mu = 0$ and any value of $\sigma^2 > 0$,

$$\frac{\sqrt{n}\bar{X}}{\sigma} \sim \mathcal{N}(0,1), \quad \frac{S^2}{\sigma^2} \sim \frac{1}{n-1}\chi^2_{n-1},$$

and these are independent. Hence the distribution of T does not depend on σ, so it is the same under all $P \in H_0$. We give this distribution a name:

Definition 3.1. *If $Z \sim \mathcal{N}(0,1)$, $U \sim \chi^2_n$, and Z and U are independent, then the distribution of $\frac{Z}{\sqrt{\frac{1}{n}U}}$ is called the t-distribution with n degrees of freedom, denoted by t_n.*

So under H_0, $T \sim t_{n-1}$. Letting $t_{n-1}(\alpha)$ denote the upper α point (or $1-\alpha$ quantile) of the distribution t_{n-1}, the test that rejects for $T > t_{n-1}(\alpha)$ is called the **one-sample t-test**.

Remark 3.2. *The one-sample t-test is often used in paired two-sample settings, such as Example 3.1.1. There, we actually have two paired samples - the before and after test scores of each student - and we perform the test by first taking the differences of these paired values. In such settings, the test is often called the* ***paired two-sample t-test****, although the statistical procedure is really just a test for one set of IID observations.*

4 Two-sample t-test and signed rank test

4.1 Two-sample t-test

Consider the setting of two independent samples $X_1, \ldots, X_n \overset{IID}{\sim} \mathcal{N}(\mu_X, \sigma^2)$ and $Y_1, \ldots, Y_m \overset{\text{IID}}{\sim} \mathcal{N}(\mu_Y, \sigma^2)$, as in Example 3.1.2. Here μ_X, μ_Y, σ^2 are all unknown; note that we are assuming (for now) a common variance σ^2 for both samples. For the testing problem

$$H_0 : \mu_X = \mu_Y$$
$$H_1 : \mu_X > \mu_Y$$

a natural idea is to reject H_0 for large values of $\bar{X} - \bar{Y}$. Observe that $\bar{X} \sim \mathcal{N}\left(\mu_X, \frac{\sigma^2}{n}\right)$, $-\bar{Y} \sim \mathcal{N}\left(-\mu_Y, \frac{\sigma^2}{m}\right)$, and these are independent. Then their sum is distributed [5] as

$$\bar{X} - \bar{Y} \sim \mathcal{N}\left(\mu_X - \mu_Y, \frac{\sigma^2}{n} + \frac{\sigma^2}{m}\right).$$

Under H_0, $\mu_X - \mu_Y = 0$, so $\frac{(\bar{X}-\bar{Y})}{\sqrt{\frac{\sigma^2}{n}+\frac{\sigma^2}{m}}} \sim \mathcal{N}(0,1)$. If σ^2 were known, then a level-α test based on $\bar{X} - \bar{Y}$ would reject when

$$\frac{\bar{X} - \bar{Y}}{\sqrt{\frac{\sigma^2}{n} + \frac{\sigma^2}{m}}} > z(\alpha).$$

Since σ^2 is unknown, we estimate it from the data. We may use both the X_i's and Y_i's to estimate σ^2 by taking the **pooled sample variance**

$$S_p^2 = \frac{1}{m+n-2}\left(\sum_{i=1}^{n}\left(X_i - \bar{X}\right)^2 + \sum_{j=1}^{m}\left(Y_j - \bar{Y}\right)^2\right),$$

[5] Recall, that if $X \sim \mathcal{N}\left(\mu_1, \sigma_1^2\right)$ and $Y \sim \mathcal{N}\left(\mu_2, \sigma_2^2\right)$ are independent, then $X + Y \sim \mathcal{N}\left(\mu_1 + \mu_2, \sigma_1^2 + \sigma_2^2\right)$.

and take as a test statistic

$$T = \frac{\bar{X} - \bar{Y}}{S_p\sqrt{\frac{1}{n} + \frac{1}{m}}}.$$

To derive the null distribution and rejection threshold for T, we may rewrite this as

$$T = \frac{\bar{X} - \bar{Y}}{\sqrt{\frac{\sigma^2}{n} + \frac{\sigma^2}{m}}} \Bigg/ \sqrt{S_p^2/\sigma^2}.$$

By Theorem 3.1 (and by independence of the two samples), $\bar{X}$, $\bar{Y}$, $\sum_i (X_i - \bar{X})^2$, $\sum_j (Y_j - \bar{Y})^2$ are all independent, with the last two quantities distributed as $\sigma^2\chi^2_{n-1}$ and $\sigma^2\chi^2_{m-1}$. Then under H_0,

$$\frac{\bar{X} - \bar{Y}}{\sqrt{\frac{\sigma^2}{n} + \frac{\sigma^2}{m}}} \sim \mathcal{N}(0,1), \quad \frac{S_p^2}{\sigma^2} \sim \frac{1}{m+n-2}\chi^2_{m+n-2},$$

and these are independent. So the distribution of T is the same for all data distributions $P \in H_0$ and is given by

$$T \sim t_{m+n-2}.$$

The test that rejects H_0 when $T > t_{m+n-2}(\alpha)$ (the upper α point of the t_{m+n-2} distribution) is called the **two-sample t-test**.

Remark 4.1. *The assumption of common variance σ^2 for the two samples is oftentimes problematic (and violated) in practice. If we assume instead that $X_1, \ldots, X_n \overset{IID}{\sim} \mathcal{N}(\mu_X, \sigma_X^2)$ and $Y_1, \ldots, Y_m \overset{IID}{\sim} \mathcal{N}(\mu_Y, \sigma_Y^2)$ for possibly different values of σ_X^2 and σ_Y^2, then $\mathrm{Var}(\bar{X} - \bar{Y}) = \frac{1}{n}\sigma_X^2 + \frac{1}{m}\sigma_Y^2$, and we may estimate this by $\frac{1}{n}S_X^2 + \frac{1}{m}S_Y^2$, where S_X^2 and S_Y^2 are the sample variances of the two samples. Then we may use the test statistic*

$$T_{welch} = \frac{\bar{X} - \bar{Y}}{\sqrt{\frac{1}{n}S_X^2 + \frac{1}{m}S_Y^2}}.$$

The distribution of T_{welch} under H_0 is no longer exactly a t distribution, but it was shown by Welch (1947)[6] to be close to the t distribution with

$$\frac{\left(S_X^2/n + S_Y^2/m\right)^2}{\left(S_X^2/n\right)^2/(n-1) + \left(S_Y^2/m\right)^2/(m-1)}$$

degrees of freedom. The test that rejects when T_{welch} exceeds the upper α point of this t distribution is called ***Welch's t-test*** *or the* ***unequal variances t-test****.*

4.2 Wilcoxon signed rank test (Non-parametric Test)

Let's return to the one-sample setting $X_1, \ldots, X_n \overset{IID}{\sim} f$, where we drop the normality assumption and only wish to test

[6] https://www.jstor.org/stable/2332510

$$\begin{aligned} &H_0 : f \text{ is symmetric about } 0 \\ &H_1 : f \text{ is symmetric about } \mu \text{ for some } \mu > 0. \end{aligned}$$

Because the shape of f is arbitrary under H_0, the distribution of the t-statistic is no longer the same under every data distribution $P \in H_0$ – in particular, it can be very far from t_{n-1} if n is moderately small and f is heavy-tailed. We consider instead the **signed rank statistic** W_+, defined in the following way:

1. Sort $|X_1|, |X_2|, \ldots, |X_n|$ in increasing order. Assign the smallest value (closest to zero) a rank of 1, the next smallest value a rank of 2, etc., and the largest value a rank of n.

2. Define W_+ as the sum of the ranks corresponding to only the positive values of $X_1, \ldots, X_n$.

As an example, suppose we have four observations $X_1 = 2, X_2 = -4, X_3 = -1, X_4 = 10$. Then, the ranks of these four observations would be $2, 3, 1, 4$. Observations X_1 and X_4 are positive, so $W_+ = 2 + 4 = 6$.

We expect W_+ to be larger under H_1 than under H_0, because high-rank observations are more likely to be positive under H_1. The test that rejects for large W_+ is called **Wilcoxon's signed rank test**. The following theorem states that W_+ has the same distribution under every $P \in H_0$, and provides a method for determining the null distribution and rejection threshold for W_+ when n is large. (When n is small, we can determine the exact null distribution of W_+ by computing W_+ for all 2^n possible combinations of $+$ and $-$ signs for the ranked data.)

Theorem 4.1. *The distribution of W_+ is the same for every PDF f that is symmetric about 0. For large n, this distribution is approximately $\mathcal{N}\left(\frac{n(n+1)}{4}, \frac{n(n+1)(2n+1)}{24}\right)$. (More formally, $\sqrt{\frac{24}{n(n+1)(2n+1)}}\left(W_+ - \frac{n(n+1)}{4}\right) \to \mathcal{N}(0,1)$ in distribution as $n \to \infty$.)*

Proof. We'll show that the distribution of W_+ is the same for every f, and that

$$\mathbb{E}\left[W_+\right] = \frac{n(n+1)}{4} \text{ and } \operatorname{Var}\left[W_+\right] = \frac{n(n+1)(2n+1)}{24}.$$

We'll provide only a heuristic explanation of why W_+ is asymptotically normal.

Let $f_0(x_1, \ldots, x_n) = \prod_{i=1}^n f(x_i)$ be the joint PDF of the data. By symmetry of f about 0, $f_0(\pm x_1, \ldots, \pm x_n)$ is the same for each of the 2^n combinations of $+/-$ signs. This implies, conditional on $|X_1|, \ldots, |X_n|$, the signs of $X_1, \ldots, X_n$ are independent and each equal to $+$ or $-$ with probability $\frac{1}{2}$. Then, letting $I_k = 1$ if the value with rank k is positive and $I_k = 0$ if it is negative, $I_1, \ldots, I_n \overset{IID}{\sim} \text{Bernoulli}\left(\frac{1}{2}\right)$ for any PDF f that is symmetric about 0.

The signed rank statistic is

$$W_+ = \sum_{k=1}^n k I_k.$$

Since $I_1, \ldots, I_n$ have the same distribution under any symmetric PDF f about 0, the distribution of W_+ is the same for all such PDFs f. We compute

$$\mathbb{E}\left[W_+\right] = \sum_{k=1}^{n} k\mathbb{E}\left[I_k\right] = \frac{1}{2}\sum_{k=1}^{n} k = \frac{n(n+1)}{4},$$
$$\operatorname{Var}\left[W_+\right] = \sum_{k=1}^{n} \operatorname{Var}\left[kI_k\right] = \sum_{k=1}^{n} k^2 \operatorname{Var}\left[I_k\right] = \frac{1}{4}\sum_{k=1}^{n} k^2 = \frac{n(n+1)(2n+1)}{24},$$

where the computation for variance uses that $I_1, \ldots, I_n$ are independent.

To explain why W_+ is approximately normally distributed, define the **empirical CDF** of $|X_1|, \ldots, |X_n|$ by

$$F_n(t) = \frac{1}{n}\sum_{i=1}^{n} \mathbb{1}\left\{|X_i| \le t\right\}.$$

($F_n(t)$ is the fraction of values of $|X_i|$ that are at most t.) Then the rank associated with X_i is exactly $nF_n\left(|X_i|\right)$, so

$$W_+ = \sum_{i=1}^{n} nF_n\left(|X_i|\right)\mathbb{1}\left\{X_i > 0\right\}.$$

When n is large, one may show that $F_n(t)$ is, with high probabiliy, close to the true CDF $F(t)$ of $|X_i|$ for every $t \in \mathbb{R}$, and hence that the difference between $W_+$and

$$\tilde{W}_+ = \sum_{i=1}^{n} nF\left(|X_i|\right)\mathbb{1}\left\{X_i > 0\right\}$$

is negligible. But $\tilde{W}$ is just the sum of IID random variables $Y_i := nF\left(|X_i|\right)\mathbb{1}\left\{X_i > 0\right\}$, and hence asymptotically normally distributed by the CLT. □

5 Tests for Variances

Continuing our development of hypothesis tests for various population parameters, in this lesson, we'll focus on hypothesis tests for population variances. Specifically, we'll develop:

- a hypothesis test for testing whether a single population variance σ^2 equals a particular value
- a hypothesis test for testing whether two population variances are equal

5.1 One Variance

The theoretical work for developing a hypothesis test for a population variance σ^2 is already behind us. Recall that if you have a random sample of size n from a normal population with (unknown) mean μ and variance σ^2, then:

$$\chi^2 = \frac{(n-1)S^2}{\sigma^2}$$

follows a chi-square distribution with $n-1$ degrees of freedom. Therefore, if we're interested in testing the null hypothesis:

$$H_0 : \sigma^2 = \sigma_0^2$$

against any of the alternative hypotheses:

$$H_A : \sigma^2 \neq \sigma_0^2, \quad H_A : \sigma^2 < \sigma_0^2, \text{ or } H_A : \sigma^2 > \sigma_0^2$$

we can use the test statistic:

$$\chi^2 = \frac{(n-1)S^2}{\sigma_0^2}$$

and follow the standard hypothesis testing procedures.

Example 5.1.1. *A manufacturer of hard safety hats for construction workers is concerned about the mean and the variation of the forces its helmets transmit to wearers when subjected to an external force. The manufacturer has designed the helmets so that the mean force transmitted by the helmets to the workers is 800 pounds (or less) with a standard deviation to be less than 40 pounds. Tests were run on a random sample of $n = 40$ helmets, and the sample mean and sample standard deviation were found to be 825 pounds and 48.5 pounds, respectively. Do the data provide sufficient evidence, at the $\alpha = 0.05$ level, to conclude that the population standard deviation exceeds 40 pounds?*

Solution: We're interested in testing the null hypothesis:

$$H_0 : \sigma^2 = 40^2 = 1600$$

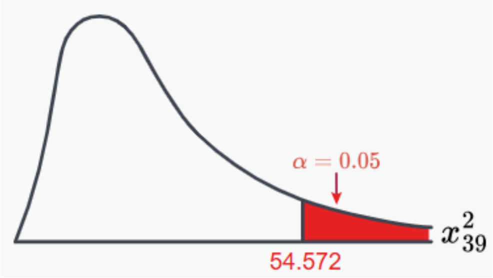
against the alternative hypothesis:

$$H_A : \sigma^2 > 1600$$

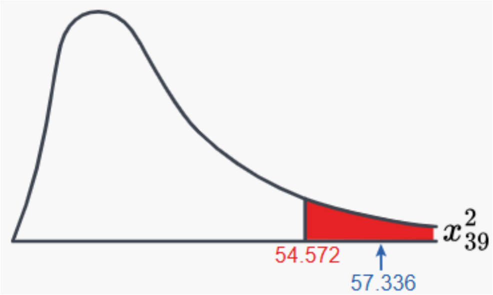
Therefore, the value of the test statistic is:

$$\chi^2 = \frac{(40-1)48.5^2}{40^2} = 57.336$$

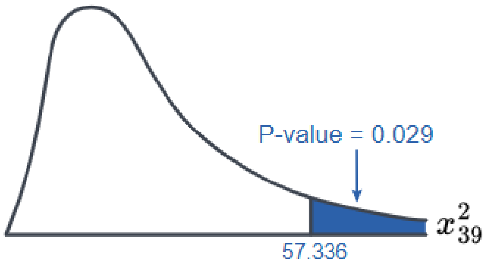
Is the test statistic too large for the null hypothesis to be true? Well, the ***critical value approach*** *would have us find the threshold value such that the probability of rejecting the null hypothesis if it were true, that is, of committing a Type I error, is 0.05, in this case. Using the chi-square probability table, we see that the cutoff value is 54.572:*



That is, we reject the null hypothesis in favor of the alternative hypothesis if the test statistic χ^2 is greater than 54.572. That is, the test statistic falls in the rejection region:



Therefore, we conclude that there is sufficient evidence, at the 0.05 level, to conclude that the population standard deviation exceeds 40.
Of course, the p-**value approach** *yields the same conclusion. In this case, the p-value is the probability that we would observe a chi-square(39) random variable more extreme than 57.336:*



As the drawing illustrates, the p-value is 0.029 (as determined using chi-square probability). Because $p = 0.029 \leq 0.05$, *we reject the null hypothesis in favor of the alternative hypothesis.*

Do the data provide sufficient evidence, at the $\alpha = 0.05$ ***level, to conclude that the population standard deviation differs from 40 pounds?***
In this case, we're interested in testing the null hypothesis:

$$H_0 : \sigma^2 = 40^2 = 1600$$

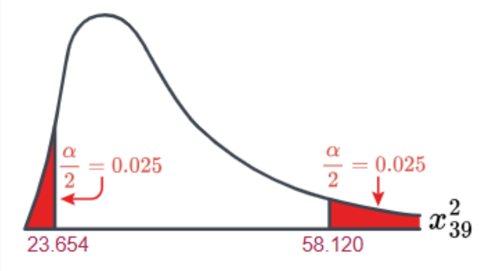
against the alternative hypothesis:

$$H_A : \sigma^2 \neq 1600$$

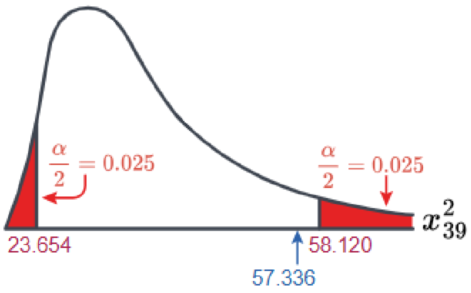
The value of the test statistic remains the same. It is again:

$$\chi^2 = \frac{(40-1)48.5^2}{40^2} = 57.336$$

Now, is the test statistic either too large or too small for the null hypothesis to be true? Well, the ***critical value approach*** *would have us dividing the significance level* $\alpha = 0.05$ *into 2, to get 0.025, and putting one of the halves in the left tail, and the other half in the other tail. Doing so we get that the lower cutoff value is 23.654 and the upper cutoff value is 58.120:*



That is, we reject the null hypothesis in favor of the two-sided alternative hypothesis if the test statistic χ^2 *is either smaller than 23.654 or greater than 58.120. It is not. That is, the test statistic does not fall in the rejection region:*



Therefore, we fail to reject the null hypothesis. There is insufficient evidence, at the 0.05 level, to conclude that the population standard deviation differs from 40. Of course, the ***p-value approach*** *again yields the same conclusion. In this case, we simply double the p-value we obtained for the one-tailed test yielding a p-value of 0.058:*

$$p = 2 \times P\left(\chi^2_{39} > 57.336\right) = 2 \times 0.029 = 0.058$$

Because $p = 0.058 > 0.05$, *we fail to reject the null hypothesis in favor of the two-sided alternative hypothesis.*

The above example illustrates an important fact, namely, that the conclusion for the one-sided test does not always agree with the conclusion for the two-sided test. If you have reason to believe that the parameter will differ from the null value in a particular direction, then you should conduct the one-sided test.

5.2 Two Variances

Let's now recall the theory necessary for developing a hypothesis test for testing the equality of two population variances. Suppose $X_1, X_2, \ldots, X_n$ is a random sample of size n from a normal population with mean μ_X and variance σ^2_X. And, suppose, independent of the first sample, $Y_1, Y_2, \ldots, Y_m$ is another random sample of size m from a normal population with μ_Y and variance σ^2_Y. Recall then, in this situation, that:

$$\frac{(n-1)S^2_X}{\sigma^2_X} \text{ and } \frac{(m-1)S^2_Y}{\sigma^2_Y}$$

have independent chi-square distributions with $n-1$ and $m-1$ degrees of freedom, respectively. Therefore:

$$F = \frac{\left[\frac{(n-1)S^2_X}{\sigma^2_x}/(n-1)\right]}{\left[\frac{(m-1)S^2_Y}{\sigma^2_Y}/(m-1)\right]} = \frac{S^2_X}{S^2_Y} \cdot \frac{\sigma^2_Y}{\sigma^2_X}$$

follows an F distribution with $n-1$ numerator degrees of freedom and $m-1$ denominator degrees of freedom. Therefore, if we're interested in testing the null hypothesis:

$$H_0 : \sigma^2_X = \sigma^2_Y \text{ (or equivalently } H_0 : \frac{\sigma^2_Y}{\sigma^2_X} = 1 \text{)}$$

against any of the alternative hypotheses:

$$H_A : \sigma^2_X \neq \sigma^2_Y, \quad H_A : \sigma^2_X > \sigma^2_Y, \text{ or } H_A : \sigma^2_X < \sigma^2_Y$$

we can use the test statistic:

$$F = \frac{S^2_X}{S^2_Y}$$

and follow the standard hypothesis testing procedures. When doing so, we might also want to recall this important fact about the F distribution:

$$F_{1-(\alpha/2)}(n-1, m-1) = \frac{1}{F_{\alpha/2}(m-1, n-1)}$$

so that when we use the critical value approach for a two-sided alternative:

$$H_A : \sigma_X^2 \neq \sigma_Y^2$$

we reject if the test statistic F is too large:

$$F \geq F_{\alpha/2}(n-1, m-1)$$

or if the test statistic F is too small:

$$F \leq F_{1-(\alpha/2)}(n-1, m-1) = \frac{1}{F_{\alpha/2}(m-1, n-1)}$$

Example 5.2.1. *A psychologist was interested in exploring whether or not male and female college students have different driving behaviors. The particular statistical question she framed was as follows: 'Is the mean fastest speed driven by male college students different than the mean fastest speed driven by female college students?' The psychologist conducted a survey of a random $n = 34$ male college students and a random $m = 29$ female college students. Here is a descriptive summary of the results of her survey:*

Males (X)	Females (Y)
$n = 34$	$m = 29$
$\bar{x} = 105.5$	$\bar{y} = 90.9$
$s_x = 20.1$	$s_y = 12.2$

Is there sufficient evidence at the $\alpha = 0.05$ level to conclude that the variance of the fastest speed driven by male college students differs from the variance of the fastest speed driven by female college students?

Solution: We're interested in testing the null hypothesis:

$$H_0 : \sigma_X^2 = \sigma_Y^2$$

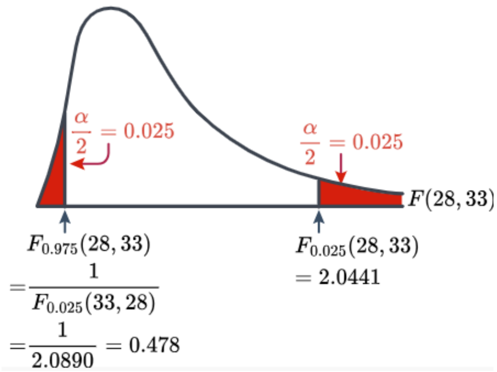
against the alternative hypothesis:

$$H_A : \sigma_X^2 \neq \sigma_Y^2$$

The value of the test statistic is:

$$F = \frac{12.2^2}{20.1^2} = 0.368$$

*Using the **critical value approach**, we divide the significance level $\alpha = 0.05$ into 2, to get 0.025, and put one of the halves in the left tail, and the other half in the other tail. Doing so, we get that the lower cutoff value is 0.478 and the upper cutoff value is 2.0441:*



Because the test statistic falls in the rejection region, that is, because $F = 0.368 \leq 0.478$, we reject the null hypothesis in favor of the alternative hypothesis. There is sufficient evidence at the $\alpha = 0.05$ level to conclude that the population variances are not equal.

6 Rank sum test and Permutation tests

6.1 Rank sum test

The idea of converting observed data values to just their ranks, so as to deal with heavy-tailed data and deviations from normality, can be extended to the two-sample setting. Consider two independent samples $X_1, \dots, X_n \overset{IID}{\sim} f$ and $Y_1, \dots, Y_m \overset{IID}{\sim} g$, where f and g are two arbitrary PDFs, and the testing problem

$$\begin{aligned} H_0 &: f = g \\ H_1 &: f \text{ stochastically dominates } g. \end{aligned}$$

(Recall from Section 3 that this alternative is one way of saying that values drawn from f "tend to be larger" than values drawn from g.)

The **rank-sum statistic** T_Y is defined as follows:

1. Consider the **pooled sample** of all observations $X_1, \dots, X_n, Y_1, \dots, Y_m$. Sort these $m + n$ values in increasing order. Assign the smallest a rank of 1, the next smallest a rank of 2, etc., and the largest a rank of $m + n$.

2. Define T_Y as the sum of the ranks corresponding to only the Y_i values, i.e., the values from only the second sample[7].

We expect T_Y to be smaller under H_1 than under H_0, because under H_1 the values of Y_i tend to have smaller ranks. The test that rejects for small values of T_Y is called the **Wilcoxon rank-sum test**, known alternatively as the Mann-Whitney U-test or the Mann-Whitney Wilcoxon test.

(If we are testing a general two-sided alternative $H_1' : f \neq g$ then we would reject for both large and small values of T_Y.)

The following theorem states that T_Y has the same distribution under every $P \in H_0$, and provides a method for determining the null distribution and rejection threshold when n and m are both large. (For

[7] One may consider equivalently T_X (the sum of ranks of the X_i's) as $T_X + T_Y$ is a fixed constant.

small n and m, we can determine the exact null distribution of T_Y by computing T_Y for all $\left(\begin{array}{c} n+m \\ m \end{array} \right)$ possible sets of ranks for the Y_i 's.)

Theorem 6.1. *The distribution of T_Y is the same under any PDF $f = g$. For large n and m, this distribution is approximately $\mathcal{N}\left(\frac{m(m+n+1)}{2}, \frac{mn(m+n+1)}{12}\right)$.*

We won't prove this result; let's just make the following comments:

- If $f = g$, then each ordering of $X_1, \ldots, X_n, Y_1, \ldots, Y_m$ is equally likely. Since T_Y depends only on this ordering, its distribution must be the same under every PDF $f = g$
- Let $I_k = 1$ if the k^{th} largest value in $X_1, \ldots, X_n, Y_1, \ldots, Y_m$ belongs to the second sample, and $I_k = 0$ otherwise. Then

$$T_Y = \sum_{k=1}^{m+n} k I_k.$$

Under H_0, I_k indicates whether the k^{th} "individual" is selected in a simple random sample of size m (without replacement) from a population of size $m+n$. Then the same computations as in Lecture 1 yield formulas for $\mathbb{E}[I_k], \mathrm{Var}[I_k]$, and $\mathrm{Cov}[I_j, I_k]$. Applying linearity of expectation and bilinearity of covariance, we may obtain

$$\mathbb{E}[T_Y] = \frac{m(m+n+1)}{2} \text{ and } \mathrm{Var}[T_Y] = \frac{mn(m+n+1)}{12}$$

as in the above theorem. (Details are provided in Rice [8], Section 11.2.3 Theorem A and Section 7.3.1 Theorems A and B.)

6.2 Permutation and randomization tests

The main idea behind the (one-sample) signed-rank test and the (two-sample) rank-sum test is to exploit a symmetry under H_0. For the signed-rank test, the symmetry is that it is equally likely to observe $\pm X_1, \ldots, \pm X_n$ for each of the 2^n combinations of $+/-$ signs. For the rank-sum test, the symmetry is that it is equally likely to observe each of the $(m+n)!$ permutations of the pooled sample $X_1, \ldots, X_n, Y_1, \ldots, Y_m$.

In fact, this idea of exploiting symmetry provides an alternative (and useful) simulation-based method of obtaining a null distribution for any test statistic T for these problems:

Example 6.2.1. *Consider two samples $X_1, \ldots, X_n$ and $Y_1, \ldots, Y_m$, and any test statistic*

$$T(X_1, \ldots, X_n, Y_1, \ldots, Y_m).$$

(For concreteness, you can think about $T = \bar{X} - \bar{Y}$) For a null hypothesis H_0 which specifies that all data from both samples are IID from a common distribution, for example

$$H_0 : X_1, \ldots, X_n, Y_1, \ldots, Y_m \overset{IID}{\sim} f$$

*for an unknown PDF f, the **permutation null distribution** of T is the distribution of*

$$T(X_1^*, \ldots, X_n^*, Y_1^*, \ldots, Y_m^*)$$

[8] Rice, John A. Mathematical statistics and data analysis. Cengage Learning, 2006.

when we fix the observed values $X_1, \ldots, X_n, Y_1, \ldots, Y_m$ and let $(X_1^*, \ldots, X_n^*, Y_1^*, \ldots, Y_m^*)$ be a permutation of $X_1, \ldots, X_n, Y_1, \ldots, Y_m$ chosen uniformly at random from the set of all $(m+n)!$ possible permutations. (For $T = \bar{X} - \bar{Y}$, what this effectively means is that we randomly choose n of the observations to be $X_1^*, \ldots, X_n^*$, set the remaining m observations to be $Y_1^*, \ldots, Y_m^*$, and compute $\bar{X}^* - \bar{Y}^*$)

Under H_0, each of these $(m+n)!$ possible values of T are equally likely to be observed. To perform a test that rejects large values of T, we may use the following procedure:

1. *Randomly permute the pooled data B times (say $B = 10000$), and compute the value of T each time.*

2. *Compute an approximate p-value as the fraction of the B simulations where we obtained a value of T larger than t_{obs}, the value for the original (unpermuted) data. (Reject at level- α if this p-value is at most α.)*

For a two-sided test that rejects for both large and small values of T, we can compute the p-value by taking the fraction of simulations where T is larger than t_{obs} or the fraction where T is smaller than t_{obs} (whichever is smaller) and multiply by 2.

This is called a **permutation test** based on T. It is an example of a **conditional test** because we are looking at the conditional distribution of the data under H_0 given the set (but not the ordering) of their values.

The utility of this idea is that it may be applied to test statistics T where we do not understand its (unconditional) distribution under H_0 and where this distribution may vary for different PDFs $f = g$. Consider the following example:

Example 6.2.2. *Let $X_1, \ldots, X_n \in \mathcal{X}$ and $Y_1, \ldots, Y_m \in \mathcal{X}$ be two random samples of "objects" (e.g. images, websites, documents) represented in some data space $\mathcal{X}$. Suppose we have a function $d(x, y)$ that measures a "distance" between any two objects $x, y \in \mathcal{X}$.*

To test whether $X_1, \ldots, X_n$ and $Y_1, \ldots, Y_m$ appear to come from the same distribution, the following might be a reasonable test statistic:

$$T_1 = \frac{2}{nm} \sum_{i=1}^{n} \sum_{j=1}^{m} d\left(X_i, Y_j\right) - \frac{1}{\binom{n}{2}} \sum_{1 \leq i < i' \leq n} d\left(X_i, X_{i'}\right) - \frac{1}{\binom{m}{2}} \sum_{1 \leq j < j' \leq m} d\left(Y_j, Y_{j'}\right).$$

In words, T_1 is twice the average distance between an object in sample 1 and an object in sample 2, minus the average distance between two objects in sample 1 and minus the average distance between two objects in sample 2. So T_1 measures whether, on average, objects from the same sample are more similar to each other than objects from different samples.

Or we might consider a "nearest-neighbors" statistic: For each of the $m + n$ data values, look at the k other data values closest to it (as measured by the distance d) and count how many of these come from the same sample as itself. Let T_2 be the average of this count across all $m + n$ data points. So T_2 measures whether the k closest other objects tend to come from the same sample.

The distributions of T_1 and T_2 under H_0 may be difficult to understand theoretically and may depend on the unknown common distribution of $X_1, \ldots, X_n, Y_1, \ldots, Y_m$, but we can still carry out a permutation test based on T_1 or on T_2.

A similar idea may be applied in the one-sample setting for testing the null hypothesis

$$H_0 : X_1, \dots, X_n \overset{\text{IID}}{\sim} f, \text{ for some PDF } f \text{ symmetric about } 0$$

based on the symmetry underlying the Wilcoxon signed-rank test.

7 Tests About Proportions

We'll start our exploration of hypothesis tests by focusing on population proportions. Specifically, we'll derive the methods used for testing:

- whether a single population proportion p equals a particular value, p_0
- whether the difference in two population proportions $p_1 - p_2$ equals a particular value p_0, say, with the most common value being 0

This allows us to test whether the proportions of the two populations are equal. Along the way, we'll learn two approaches to hypothesis testing: the critical value approach and the p-value approach.

7.1 Testing Single Proportion

Every time we perform a hypothesis test, this is the basic procedure that we will follow:

1. We'll make an initial assumption about the population parameter.
2. We'll collect evidence or else use somebody else's evidence (in either case, our evidence will come in the form of data).
3. Based on the available evidence (data), we'll decide whether to "**reject**" or "**not reject**" our initial assumption.

Example 7.1.1. *A four-sided (tetrahedral) die is tossed 1000 times, and 290 fours are observed. Is there evidence to conclude that the die is biased, that is, say, that more fours than expected are observed?*

Solution: As the basic hypothesis testing procedure outlines above, the first step involves stating an initial assumption that the die is unbiased. If the die is unbiased, then each side (1,2,3, and 4) is equally likely. So, we'll assume that p, the probability of getting a 4 is 0.25. In general, the initial assumption is called the null hypothesis and is denoted H_0. (That's a zero in the subscript for 'null'). In statistical notation, we write the initial assumption as:

$$H_0 : p = 0.25$$

That is, the initial assumption involves making a statement about a population proportion. Now, the second step tells us that we need to collect evidence (data) for or against our initial assumption. In this case, that's already been done for us. We were told that the die was tossed $n = 1000$ times, and $y = 290$ fours were observed. Using statistical notation again, we write the collected evidence as a sample proportion:

$$\hat{p} = \frac{y}{n} = \frac{290}{1000} = 0.29$$

Now we just need to complete the third step of deciding whether or not to reject our initial assumption that the population proportion is 0.25. Recall that the Central Limit Theorem tells us that the sample proportion:

$$\hat{p} = \frac{Y}{n}$$

is approximately normally distributed with (assumed) mean:

$$p_0 = 0.25$$

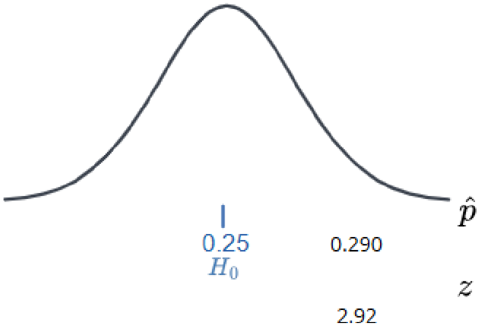
and (assumed) standard deviation:

$$\sqrt{\frac{p_0\,(1-p_0)}{n}} = \sqrt{\frac{0.25(0.75)}{1000}} = 0.01369$$

That means that:

$$Z = \frac{\hat{p} - p_0}{\sqrt{\frac{p_0(1-p_0)}{n}}}$$

follows a standard normal $N(0,1)$ distribution. So, we can 'translate' our observed sample proportion of 0.290 onto the Z scale. Here's a picture that summarizes the situation:



So, we are assuming that the population proportion is 0.25 (in blue), but we've observed a sample proportion of 0.290 that falls way out in the right tail of the normal distribution. It certainly doesn't appear impossible to obtain a sample proportion of 0.29. But, that's what we're left with to decide. That is, we have to decide if a sample proportion of 0.290 is more extreme than we'd expect if the population proportion p does indeed equal 0.25.

There are two approaches to making the decision:

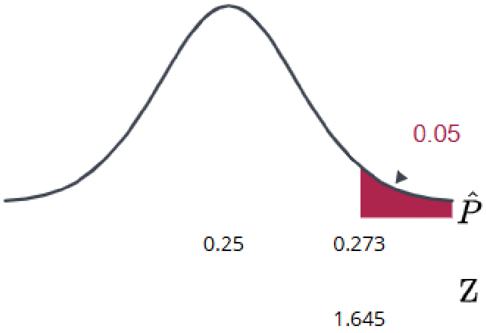
1. *one is called the* ***'critical value'*** *(or 'critical region' or 'rejection region') approach*

2. *and the other is called the* ***'p-value'*** *approach*

We probably wouldn't reject our initial assumption that the population proportion $p = 0.25$ if our observed sample proportion were 0.255. And, we might still not be inclined to reject our initial assumption that the population proportion $p = 0.25$ if our observed sample proportion were 0.27. On the other hand, we would almost certainly want to reject our initial assumption that the population proportion $p = 0.25$ if our observed sample proportion were 0.35. That suggests, then, that there is some 'threshold' value that once we 'cross' the threshold value, we are inclined to reject our initial assumption. That is the critical value approach in a nutshell. That is, the critical value approach tells us to define a threshold value, called a 'critical value' so that if our 'test statistic' is more extreme than the critical value, then *we reject the null hypothesis. Let's suppose that we decide to reject the null hypothesis* $H_0 : p = 0.25$ *in favor of the 'alternative hypothesis'* $H_A : p > 0.25$ *if:*

$$\hat{p} > 0.273 \textit{ or equivalently if } Z > 1.645$$

Here's a picture of such a 'critical region' (or 'rejection region'):



Note, by the way, that the 'size' of the critical region is 0.05. This will become apparent in a bit when we talk below about the possible errors that we can make whenever we conduct a hypothesis test. At any rate, let's get back to deciding whether our particular sample proportion appears to be too extreme. Well, it looks like we should reject the null hypothesis (our initial assumption $p = 0.25$*) because:*

$$\hat{p} = 0.29 > 0.273$$

or equivalently since our test statistic:

$$Z = \frac{\hat{p} - p_0}{\sqrt{\frac{p_0(1-p_0)}{n}}} = \frac{0.29 - 0.25}{\sqrt{\frac{0.25(0.75)}{1000}}} = 2.92$$

is greater than 1.645. Our conclusion: we say there is sufficient evidence to conclude $H_A : p > 0.25$*, that is, that the die is biased. By the way, this example involves what is called a one-tailed test, or more specifically, a right-tailed test, because the critical region falls in only one of the two tails of the normal distribution, namely the right tail.*

Let's revisit the basic hypothesis testing procedure that we outlined above. This time, though, let's state the procedure in terms of performing a hypothesis test for a population proportion using the critical value approach. The basic procedure is:

1. State the null hypothesis H_0 and the alternative hypothesis H_A. (By the way, some textbooks, including ours, use the notation H_1 instead of H_A to denote the alternative hypothesis.)

2. Calculate the test statistic:
$$Z = \frac{\hat{p} - p_0}{\sqrt{\frac{p_0(1-p_0)}{n}}}.$$

3. Determine the critical region.

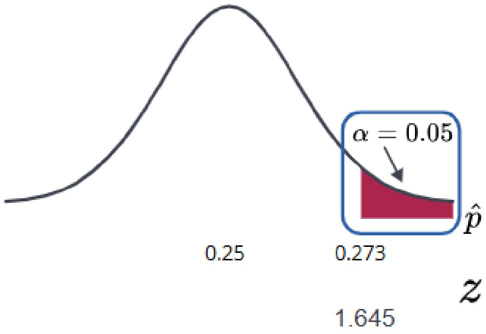
4. Make a decision. Determine if the test statistic falls in the critical region. If it does, reject the null hypothesis. If it does not, do not reject the null hypothesis.

Now, back to those possible errors we can make when conducting such a hypothesis test.

Possible Errors

Every time we conduct a hypothesis test, we have a chance of making an error.

1. If we reject the null hypothesis H_0 (in favor of the alternative hypothesis H_A) when the null hypothesis is in fact true, we say we've committed a Type I error. For our example above, we set P (Type I error) equal to 0.05:

   That's why the 0.05! We wanted to minimize our chance of making a Type I error! In general, we denote $\alpha = P$ (Type I error) = the 'significance level of the test'. We want to minimize α. Therefore, typical α values are 0.01, 0.05, and 0.10.

2. If we fail to reject the null hypothesis when the null hypothesis is false, we say we've committed a Type II error. For our example, suppose (unknown to us) that the population proportion p is 0.27. Then, the probability of a Type II error, in this case, is:

   $$P(\text{ Type II Error }) = P(\hat{p} < 0.273 \text{ if } p = 0.27) = P\left(Z < \frac{0.273 - 0.27}{\sqrt{\frac{0.27(0.73)}{1000}}}\right) = P(Z < 0.214) = 0.5847$$

   In general, we denote $\beta = P$ (Type II error). Just as we want to minimize $\alpha = P$ (Type I error), we want to minimize $\beta = P(\text{ Type II error })$. Typical β values are $0.05, 0.10$, and 0.20.

Example 7.1.2. *Let p equal the proportion of drivers who use a seat belt in a state that does not have a mandatory seat belt law. It was claimed that $p = 0.14$. An advertising campaign was conducted to increase this proportion. Two months after the campaign, $y = 104$ out of a random sample of $n = 590$ drivers were wearing seat belts. Was the campaign successful?*

Solution: The observed sample proportion is:

$$\hat{p} = \frac{104}{590} = 0.176$$

Because it is claimed that $p = 0.14$, the null hypothesis is:

$$H_0 : p = 0.14$$

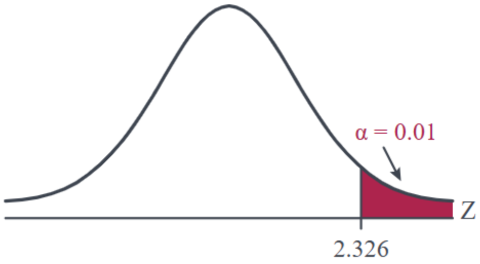
Because we're interested in seeing if the advertising campaign was successful, that is, that a greater proportion of people wear seat belts, the alternative hypothesis is:

$$H_A : p > 0.14$$

The test statistic is therefore:

$$Z = \frac{\hat{p} - p_0}{\sqrt{\frac{p_0(1-p_0)}{n}}} = \frac{0.176 - 0.14}{\sqrt{\frac{0.14(0.86)}{590}}} = 2.52$$

If we use a significance level of $\alpha = 0.01$, then the critical region is:



That is, we reject the null hypothesis if the test statistic $Z > 2.326$. Because the test statistic falls in the critical region, that is, because $Z = 2.52 > 2.326$, we can reject the null hypothesis in favor of the alternative hypothesis. There is sufficient evidence at the $\alpha = 0.01$ level to conclude the campaign was successful ($p > 0.14$). Again, note that this is an example of a right-tailed hypothesis test because the action falls in the right tail of the normal distribution.

Example 7.1.3. *A Gallup poll released on October 13, 2000, found that 47% of the 1052 U.S. adults surveyed classified themselves as 'very happy' when given the choices of:*

1. *'very happy'*
2. *'fairly happy'*
3. *'not too happy'*

Suppose that a journalist who is a pessimist took advantage of this poll to write a headline titled "Poll finds that U.S. adults who are very happy are in the minority". Is the pessimistic journalist's headline warranted?

Solution: The sample proportion is:

$$\hat{p} = 0.47$$

Because we're interested in the majority/minority boundary line, the null hypothesis is:

$$H_0 : p = 0.50$$

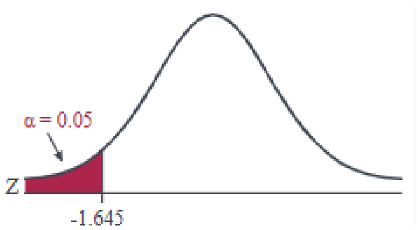
Because the journalist claims that the proportion of very happy U.S. adults is a minority, that is, less than 0.50, the alternative hypothesis is:

$$H_A : p < 0.50$$

The test statistic is therefore:

$$Z = \frac{\hat{p} - p_0}{\sqrt{\frac{p_0(1-p_0)}{n}}} = \frac{0.47 - 0.50}{\sqrt{\frac{0.50(0.50)}{1052}}} = -1.946$$

Now, this time, we need to put our critical region in the left tail of the normal distribution. If we use a significance level of $\alpha = 0.05$, then the critical region is:



That is, we reject the null hypothesis if the test statistic $Z < -1.645$. Because the test statistic falls in the critical region, that is, because $Z = -1.946 < -1.645$, we can reject the null hypothesis in favor of the alternative hypothesis. There is sufficient evidence at the $\alpha = 0.05$ level to conclude that $p < 0.50$, that is, U.S. adults who are very happy are in the minority. The journalist's pessimism appears to be indeed warranted. Note that this is an example of a left-tailed hypothesis test because the action falls in the left tail of the normal distribution.

Up until now, we have used the critical region approach in conducting our hypothesis tests. Now, let's take a look at an example in which we use what is called the p-value approach.

Example 7.1.4. *Among patients with lung cancer, usually, 90% or more die within three years. As a result of new forms of treatment, it is felt that this rate has been reduced. In a recent study of $n = 150$ lung cancer patients, $y = 128$ died within three years. Is there sufficient evidence at the $\alpha = 0.05$ level, say, to conclude that the death rate due to lung cancer has been reduced?*

Solution: The sample proportion is:

$$\hat{p} = \frac{128}{150} = 0.853$$

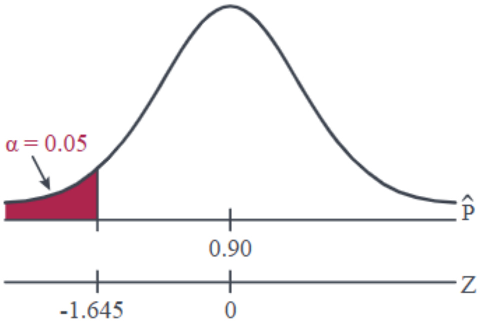
The null and alternative hypotheses are:

$$H_0 : p = 0.90 \text{ and } H_A : p < 0.90$$

The test statistic is, therefore:

$$Z = \frac{\hat{p} - p_0}{\sqrt{\frac{p_0(1-p_0)}{n}}} = \frac{0.853 - 0.90}{\sqrt{\frac{0.90(0.10)}{150}}} = -1.92$$

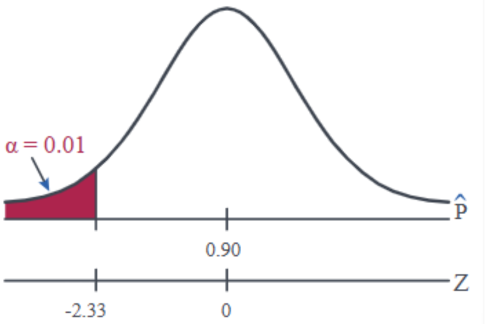
And, the rejection region is:



Since the test statistic $Z = -1.92 < -1.645$, we reject the null hypothesis. There is sufficient evidence at the $\alpha = 0.05$ level to conclude that the rate has been reduced.

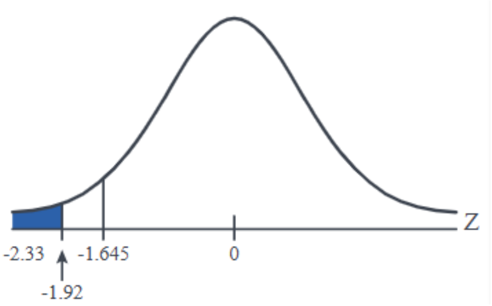
What if we set the significance level $\alpha = P$ (Type I Error) to 0.01? Is there still sufficient evidence to conclude that the death rate due to lung cancer has been reduced?

In this case, with $\alpha = 0.01$, the rejection region is $Z \leq -2.33$. That is, we reject if the test statistic falls in the rejection region defined by $Z \leq -2.33$:



Because the test statistic $Z = -1.92 > -2.33$, we do not reject the null hypothesis. There is insufficient evidence at the $\alpha = 0.01$ level to conclude that the rate has been reduced.

In the first part of this example, we rejected the null hypothesis when $\alpha = 0.05$. And, in the second part of this example, we failed to reject the null hypothesis when $\alpha = 0.01$. There must be some level of α, then, in which we cross the threshold from rejecting to not rejecting the null hypothesis. What is the smallest α-level that would still cause us to reject the null hypothesis?
We would, of course, reject any time the critical value was smaller than our test statistic -1.92:



That is, we would reject if the critical value were $-1.645, -1.83$, and -1.92. But, we wouldn't reject if the critical value were -1.93. The α-level associated with the test statistic -1.92 is called the p-value. It is the smallest α-level that would lead to rejection. In this case, the p-value is:

$$P(Z < -1.92) = 0.0274$$

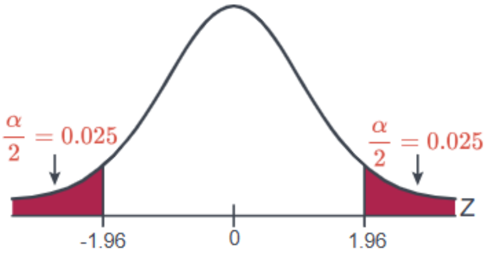
So far, all of the examples we've considered have involved a one-tailed hypothesis test in which the alternative hypothesis involved either a less than ($<$) or a greater than ($>$) sign. What happens if we aren't sure of the direction in which the proportion could deviate from the hypothesized null value? That is, what if the alternative hypothesis involved a not-equal sign ($\neq$)? Let's take a look at an example.

Example 7.1.5. *What if we wanted to perform a 'two-tailed' test? That is, what if we wanted to test:*

$$H_0 : p = 0.90 \text{ versus } H_A : p \neq 0.90$$

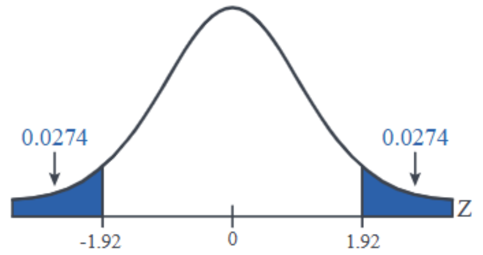
at the $\alpha = 0.05$ level?

Solution: Let's first consider the critical value approach. If we allow for the possibility that the sample proportion could either prove to be too large or too small, then we need to specify a threshold value, that *is, a critical value, in each tail of the distribution. In this case, we divide the 'significance level'* α *by 2 to get* $\alpha/2$:



That is, our rejection rule is that we should reject the null hypothesis H_0 *if* $Z \geq 1.96$ *or we should reject the null hypothesis* H_0 *if* $Z \leq -1.96$. *Alternatively, we can write that we should reject the null hypothesis* H_0 *if* $|Z| \geq 1.96$. *Because our test statistic is -1.92, we just barely fail to reject the null hypothesis, because* $1.92 < 1.96$. *In this case, we would say that there is insufficient evidence at the* $\alpha = 0.05$ *level to conclude that the sample proportion differs significantly from 0.90.*

Now for the p-value approach. Again, needing to allow for the possibility that the sample proportion is either too large or too small, we multiply the p-value we obtain for the one-tailed test by 2:



That is, the p-value is:

$$p = P(|Z| \geq 1.92) = P(Z > 1.92 \text{ or } Z < -1.92) = 2 \times 0.0274 = 0.055$$

Because the p-value 0.055 is (just barely) greater than the significance level $\alpha = 0.05$, *we barely fail to reject the null hypothesis. Again, we would say that there is insufficient evidence at the* $\alpha = 0.05$ *level to conclude that the sample proportion differs significantly from 0.90.*

Let's close this example by formalizing the definition of a p-value, as well as summarizing the p-value approach to conducting a hypothesis test.

p-value

The p-value is the smallest significance level α that leads us to reject the null hypothesis. Alternatively (and the way I prefer to think of p-values), the p-value is the probability that we'd observe a more extreme statistic than we did if the null hypothesis were true. If the p-value is small, that is, if $p \leq \alpha$, then we reject the null hypothesis H_0.

Remark 7.1. *By the way, to test* $H_0 : p = p_0$, *some statisticians will use the test statistic:*

$$Z = \frac{\hat{p} - p_0}{\sqrt{\frac{\hat{p}(1-\hat{p})}{n}}}$$

rather than the one we've been using:

$$Z = \frac{\hat{p} - p_0}{\sqrt{\frac{p_0(1-p_0)}{n}}}$$

One advantage of doing so is that the interpretation of the confidence interval - does it contain p_0? - is always consistent with the hypothesis test decision, as illustrated here:

For the sake of ease, let:

$$se(\hat{p}) = \sqrt{\frac{\hat{p}(1-\hat{p})}{n}}$$

Two-tailed test. *In this case, the critical region approach tells us to reject the null hypothesis $H_0 : p = p_0$ against the alternative hypothesis $H_A : p \neq p_0$:*

$$\textit{if } Z = \frac{\hat{p} - p_0}{se(\hat{p})} \geq z_{\alpha/2} \textit{ or if } Z = \frac{\hat{p} - p_0}{se(\hat{p})} \leq -z_{\alpha/2}$$

which is equivalent to rejecting the null hypothesis:

$$\textit{if } \hat{p} - p_0 \geq z_{\alpha/2}\, \text{se}(\hat{p}) \textit{ or if } \hat{p} - p_0 \leq -z_{\alpha/2}\, \text{se}(\hat{p})$$

which is equivalent to rejecting the null hypothesis:

$$\textit{if } p_0 \geq \hat{p} + z_{\alpha/2} se(\hat{p}) \textit{ or if } p_0 \leq \hat{p} - z_{\alpha/2} se(\hat{p})$$

That's the same as saying that we should reject the null hypothesis H_0 if p_0 is not in the $(1-\alpha)100\%$ confidence interval!

Left-tailed test. *In this case, the critical region approach tells us to reject the null hypothesis $H_0 : p = p_0$ against the alternative hypothesis $H_A : p < p_0$:*

$$\textit{if } Z = \frac{\hat{p} - p_0}{\text{se}(\hat{p})} \leq -z_\alpha$$

which is equivalent to rejecting the null hypothesis:

$$\textit{if } \hat{p} - p_0 \leq -z_\alpha se(\hat{p})$$

which is equivalent to rejecting the null hypothesis:

$$\textit{if } p_0 \geq \hat{p} + z_\alpha\, \text{se}(\hat{p})$$

That's the same as saying that we should reject the null hypothesis H_0 if p_0 is not in the upper $(1-\alpha)100\%$ confidence interval:

$$(0, \hat{p} + z_\alpha se(\hat{p}))$$

7.2 Comparing Two Proportions

So far, all of our examples involved testing whether a single population proportion p equals some value p_0. Now, let's turn our attention for a bit towards testing whether one population proportion p_1 equals a second population proportion p_2. Additionally, most of our examples thus far have involved left-tailed tests in which the alternative hypothesis involved $H_A : p < p_0$ or right-tailed tests in which the alternative hypothesis involved $H_A : p > p_0$. Here, let's consider an example that tests the equality of two proportions against the alternative that they are not equal. Using statistical notation, we'll test:

$$H_0 : p_1 = p_2 \text{ versus } H_A : p_1 \neq p_2$$

Theorem 7.1. *The test statistic for testing the difference in two population proportions, that is, for testing the null hypothesis $H_0 : p_1 - p_2 = 0$ is:*

$$Z = \frac{(\hat{p}_1 - \hat{p}_2) - 0}{\sqrt{\hat{p}(1-\hat{p})\left(\frac{1}{n_1} + \frac{1}{n_2}\right)}}$$

where:

$$\hat{p} = \frac{Y_1 + Y_2}{n_1 + n_2}$$

the proportion of 'successes' in the two samples combined, n_1, n_2 represents the number of samples drawn from the two populations, and Y_1, Y_2 represents the number of 'successes' in the two populations.

Proof. Recall that:

$$\hat{p}_1 - \hat{p}_2$$

is approximately normally distributed with mean:

$$p_1 - p_2$$

and variance:

$$\frac{p_1\,(1-p_1)}{n_1} + \frac{p_2\,(1-p_2)}{n_2}$$

But, if we assume that the null hypothesis is true, then the population proportions equal some common value p, say, that is, $p_1 = p_2 = p$. In that case, then the variance becomes:

$$p(1-p)\left(\frac{1}{n_1} + \frac{1}{n_2}\right)$$

So, under the assumption that the null hypothesis is true, we have that:

$$Z = \frac{(\hat{p}_1 - \hat{p}_2) - \overbrace{(p_1 - p_2)}^{0}}{\sqrt{p(1-p)\left(\frac{1}{n_1} + \frac{1}{n_2}\right)}}$$

follows (at least approximately) the standard normal $N(0,1)$ distribution. Since we don't know the (assumed) common population proportion p any more than we know the proportions p_1 and p_2 of each population, we can estimate p using:

$$\hat{p} = \frac{Y_1 + Y_2}{n_1 + n_2}$$

the proportion of 'successes' in the two samples combined. And, hence, our test statistic becomes:

$$Z = \frac{(\hat{p}_1 - \hat{p}_2) - 0}{\sqrt{\hat{p}(1-\hat{p})\left(\frac{1}{n_1} + \frac{1}{n_2}\right)}}$$

as was to be proved. □

Example 7.2.1. *Time magazine reported the result of a telephone poll of 800 adult Americans. The question posed to the Americans who were surveyed was: 'Should the federal tax on cigarettes be raised to pay for health care reform?' The results of the survey were:*

Non-Smokers	Smokers
$n_1 = 605$ $y_1 = 351$ said 'yes' $\hat{p}_1 = \frac{351}{605} = 0.58$	$n_2 = 195$ $y_2 = 41$ said 'yes' $\hat{p}_2 = \frac{41}{195} = 0.21$

Is there sufficient evidence at the $\alpha = 0.05$, say, to conclude that the two populations - smokers and non-smokers - differ significantly concerning their opinions?

Solution: If $p_1 =$ the proportion of the non-smoker population who reply 'yes' and $p_2 =$ the proportion of the smoker population who reply 'yes', then we are interested in testing the null hypothesis:

$$H_0 : p_1 = p_2$$

against the alternative hypothesis:

$$H_A : p_1 \neq p_2$$

The overall sample proportion is:

$$\hat{p} = \frac{41 + 351}{195 + 605} = \frac{392}{800} = 0.49$$

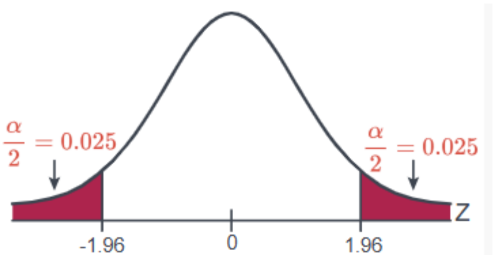
That implies then that the test statistic for testing:

$$H_0 : p_1 = p_2 \textit{ versus } H_0 : p_1 \neq p_2$$

is:

$$Z = \frac{(0.58 - 0.21) - 0}{\sqrt{0.49(0.51)\left(\frac{1}{195} + \frac{1}{605}\right)}} = 8.99$$

that Z-value is off the charts, so to speak. Let's go through the formalities anyway making the decision first using the rejection region approach, and then using the p-value approach. Putting half of the rejection region in each tail, we have:



That is, we reject the null hypothesis H_0 if $Z \geq 1.96$ or if $Z \leq -1.96$. We clearly reject H_0, since 8.99 falls in the 'red zone', that is, 8.99 is (much) greater than 1.96. There is sufficient evidence at the 0.05 level to conclude that the two populations differ with respect to their opinions concerning imposing a federal tax to help pay for health care reform.

Now for the p-value approach: That is, the p-value is less than 0.0001. Because $P < 0.0001 \leq \alpha = 0.05$, we reject the null hypothesis. Again, there is sufficient evidence at the 0.05 level to conclude that

the two populations differ with respect to their opinions concerning imposing a federal tax to help pay for health care reform.
Thankfully, as should always be the case, the two approaches - the critical value approach and the p-value approach lead to the same conclusion

Remark 7.2. *For testing $H_0 : p_1 = p_2$, some statisticians use the test statistic:*

$$Z = \frac{(\hat{p}_1 - \hat{p}_2) - 0}{\sqrt{\frac{\hat{p}_1(1-\hat{p}_1)}{n_1} + \frac{\hat{p}_2(1-\hat{p}_2)}{n_2}}}$$

instead of the one we used:

$$Z = \frac{(\hat{p}_1 - \hat{p}_2) - 0}{\sqrt{\hat{p}(1-\hat{p})\left(\frac{1}{n_1} + \frac{1}{n_2}\right)}}$$

An advantage of doing so is again that the interpretation of the confidence interval — does it contain 0? - is always consistent with the hypothesis test decision.

8 Hypothesis Testing for Categorical Data

Here, we introduce the generalized likelihood ratio test (GLRT) and explore applications to the analysis of categorical data.

8.1 GLRT for a simple null hypothesis

Let $\{f(x \mid \theta) : \theta \in \Omega\}$ be a parameteric model, and let $\theta_0 \in \Omega$ be a particular parameter value. For testing

$$H_0 : \theta = \theta_0$$
$$H_1 : \theta \neq \theta_0$$

the **generalized likelihood ratio test (GLRT)** rejects for small values of the test statistic

$$\Lambda = \frac{\operatorname{lik}(\theta_0)}{\max_{\theta \in \Omega} \operatorname{lik}(\theta)},$$

where $\operatorname{lik}(\theta)$ is the likelihood function. (In the case of IID samples $X_1, \ldots, X_n \overset{IID}{\sim} f(x \mid \theta)$, $\operatorname{lik}(\theta) = \prod_{i=1}^n f(X_i \mid \theta)$.) The numerator is the value of the likelihood at θ_0, and the denominator is the value of the likelihood at the MLE $\hat{\theta}$. The level- α GLRT rejects H_0 when $\Lambda \leq c$, where (as usual) c is chosen so that $\mathbb{P}_{H_0}[\Lambda \leq c]$ equals (or approximately equals) α.

Note that the GLRT differs from the likelihood ratio test discussed previously in the context of the Neyman-Pearson lemma, where the denominator was instead given by $\operatorname{lik}(\theta_1)$ for a simple alternative $\theta = \theta_1$. The alternative H_1 above is not simple, and the GLRT replaces the denominator with the maximum value of the likelihood over all values of θ.

Example 8.1.1. *Let $X_1, \ldots, X_n \overset{IID}{\sim} \mathcal{N}(\theta, 1)$ and consider the problem of testing*

$$H_0 : \theta = 0$$
$$H_1 : \theta \neq 0.$$

The MLE for θ is $\hat{\theta} = \bar{X}$. We compute

$$\operatorname{lik}(0) = \prod_{i=1}^{n} \frac{1}{\sqrt{2\pi}} e^{-\frac{X_i^2}{2}}$$

$$\max_{\theta \in \mathbb{R}} \operatorname{lik}(\theta) = \operatorname{lik}(\hat{\theta}) = \prod_{i=1}^{n} \frac{1}{\sqrt{2\pi}} e^{-\frac{(X_i - \bar{X})^2}{2}}.$$

Then

$$\Lambda = \frac{\operatorname{lik}(0)}{\max_{\theta \in \mathbb{R}} \operatorname{lik}(\theta)} = \exp\left(-\sum_{i=1}^{n} \frac{X_i^2}{2} + \sum_{i=1}^{n} \frac{\left(X_i - \bar{X}\right)^2}{2}\right)$$

$$= \exp\left(-\sum_{i=1}^{n} \frac{X_i^2}{2} + \sum_{i=1}^{n} \frac{X_i^2 - 2X_i\bar{X} + \bar{X}^2}{2}\right) = \exp\left(-\frac{n}{2}\bar{X}^2\right).$$

Rejecting for small values of Λ is the same as rejecting for large values of $-2\log\Lambda = n\bar{X}^2$. Under $H_0, \sqrt{n}\bar{X} \sim \mathcal{N}(0,1)$, so $n\bar{X}^2 \sim \chi_1^2$. Then the GLRT rejects H_0 when $n\bar{X}^2 > \chi_1^2(\alpha)$, the upper-$\alpha$ point of the χ_1^2 distribution. (This is the same as rejecting when $|\bar{X}| > z(\alpha/2)/\sqrt{n}$, so the GLRT is equivalent to usual two-sided z-test based on $\bar{X}$.)

In general, the exact sampling distribution of $-2\log\Lambda$ under H_0 may not have a simple form as in the above example, but it may be approximated by a chi-squared distribution for large n:

Theorem 8.1. *Let $\{f(x \mid \theta) : \theta \in \Omega\}$ be a parametric model and let $X_1, \ldots, X_n \overset{IID}{\sim} f(x \mid \theta_0)$. Suppose θ_0 is an interior point of Ω, and the regularity conditions of Theorems 1.1 and 2.1 of Chapter 2 (for consistency and asymptotic normality of the MLE) hold. Then*

$$-2\log\Lambda \to \chi_k^2$$

in distribution as $n \to \infty$, where $k = \dim\Omega$ is the dimension of Ω.

Hint. For simplicity, we consider only the case $k = 1$, so θ is a single parameter. Letting $l(\theta)$ denote the log-likelihood function and $\hat{\theta}$ denote the MLE,

$$-2\log\Lambda = -2l\left(\theta_0\right) + 2l(\hat{\theta}).$$

Applying a Taylor expansion of $l\left(\theta_0\right)$ around $\theta_0 = \hat{\theta}$,

$$l\left(\theta_0\right) \approx l(\hat{\theta}) + \left(\theta_0 - \hat{\theta}\right) l'(\hat{\theta}) + \frac{1}{2}\left(\theta_0 - \hat{\theta}\right)^2 l''(\hat{\theta}) \approx l(\hat{\theta}) - \frac{1}{2} nI\left(\theta_0\right)\left(\theta_0 - \hat{\theta}\right)^2,$$

where the second approximation uses $l'(\hat{\theta}) = 0$ and $l''(\hat{\theta}) \approx -nI(\hat{\theta}) \approx -nI\left(\theta_0\right)$. Then

$$-2\log\Lambda \approx nI\left(\theta_0\right)\left(\theta_0 - \hat{\theta}\right)^2.$$

$\sqrt{nI\left(\theta_0\right)}\left(\hat{\theta} - \theta_0\right) \to \mathcal{N}(0,1)$ in distribution by asymptotic normality of the MLE, so the continuous mapping theorem implies $-2\log\Lambda \approx nI\left(\theta_0\right)\left(\theta_0 - \hat{\theta}\right)^2 \to \chi_1^2$ as desired. □

This theorem implies that an approximate level-α test is given by rejecting H_0 when $-2\log\Lambda > \chi_k^2(\alpha)$, the upper- α point of the χ_k^2 distribution. The "dimension" k of Ω is the number of free parameters in the model or the number of parameters minus the number of independent constraints. For instance, in Example 8.1.1, there is a single parameter θ, so the dimension is 1. For a multinomial model with parameters $(p_1, \ldots, p_k)$, there are k parameters, but they are constrained to sum to 1, so the dimension is $k-1$.

8.2 GLRT for testing a sub-model

More generally, let $\Omega_0 \subset \Omega$ be a subset of the parameter space Ω, corresponding to a lower-dimensional sub-model. For testing

$$H_0 : \theta \in \Omega_0$$
$$H_1 : \theta \notin \Omega_0$$

the generalized likelihood ratio statistic is defined as

$$\Lambda = \frac{\max_{\theta\in\Omega_0} \operatorname{lik}(\theta)}{\max_{\theta\in\Omega} \operatorname{lik}(\theta)}.$$

In other words, Λ is the ratio of the values of the likelihood function evaluated at the MLE in the sub-model and at the MLE in the full-model.

For large n, under any $\theta_0 \in \Omega_0$, $-2\log\Lambda$ is approximately distributed as χ_k^2 where k is the difference in dimensionality between Ω_0 and Ω, and an approximate level-α test rejects H_0 when $-2\log\Lambda > \chi_k^2(\alpha)$:

Theorem 8.2. *Let $\{f(x \mid \theta) : \theta \in \Omega\}$ be a parametric model, and let $X_1, \ldots, X_n \overset{IID}{\sim} f(x \mid \theta_0)$ where $\theta_0 \in \Omega_0$. Suppose θ_0 is an interior point of both Ω_0 and Ω, and the regularity conditions of Theorems 1.1 and 2.1 of Chapter 3 hold for both the full model $\{f(x \mid \theta) : \theta \in \Omega\}$ and the sub-model $\{f(x \mid \theta) : \theta \in \Omega_0\}$. Then*

$$-2\log\Lambda \to \chi_k^2$$

in distribution as $n \to \infty$, where $k = \dim\Omega - \dim\Omega_0$.

Example 8.2.1. *(Hardy-Weinberg equilibrium[9]). At a single diallelic locus in the genome with two possible alleles* A *and* a*, any individual can have genotype AA, Aa, or aa. If we randomly select n individuals from a population, we may model the numbers of individuals with these genotypes as $(N_{AA}, N_{Aa}, N_{aa}) \sim \text{Multinomial}\left(n, (p_{AA}, p_{Aa}, p_{aa})\right)$.*

When the alleles A *and* a *are present in the population with proportions θ and $1-\theta$, then under an assumption of random mating, quantitative genetics theory predicts that p_{AA}, p_{Aa}, and p_{aa} should be given by $p_{AA} = \theta^2, p_{Aa} = 2\theta(1-\theta)$, and $p_{aa} = (1-\theta)^2$-this is called the Hardy-Weinberg equilibrium. In practice we do not know θ, but we may still test the null hypothesis that Hardy-Weinberg equilibrium holds for some θ:*

$$H_0 : p_{AA} = \theta^2,\ p_{Aa} = 2\theta(1-\theta),\ p_{aa} = (1-\theta)^2 \text{ for some } \theta \in (0,1).$$

This null hypothesis corresponds to a 1-dimensional sub-model (with a single free parameter θ) inside the 2-dimensional multinomial model (specified by general parameters p_{AA}, p_{Aa}, p_{aa} summing to 1). We may test H_0 using the GLRT:

[9] Read more: https://www.nature.com/scitable/knowledge/library/the-hardy-weinberg-principle-13235724/

Figure 2: The Hardy-Weinberg theorem characterizes the distributions of genotype frequencies in populations that are not evolving, and is thus the fundamental null model for population genetics.

The multinomial likelihood is given by

$$l\left(p_{AA}, p_{Aa}, p_{aa}\right) = \left(\begin{array}{c} n \\ N_{AA}, N_{Aa}, N_{aa} \end{array}\right) p_{AA}^{N_{AA}} p_{Aa}^{N_{Aa}} p_{aa}^{N_{aa}}.$$

Letting $\hat{p}_{AA}, \hat{p}_{Aa}, \hat{p}_{aa}$ *denote the full-model MLEs and* $\hat{p}_{0,AA}, \hat{p}_{0,Aa}, \hat{p}_{0,aa}$ *denote the sub-model MLEs, the generalized likelihood ratio is*

$$\Lambda = \left(\frac{\hat{p}_{0,AA}}{\hat{p}_{AA}}\right)^{N_{AA}} \left(\frac{\hat{p}_{0,Aa}}{\hat{p}_{Aa}}\right)^{N_{Aa}} \left(\frac{\hat{p}_{0,aa}}{\hat{p}_{aa}}\right)^{N_{aa}},$$

so

$$-2\log\Lambda = 2N_{AA}\log\frac{\hat{p}_{AA}}{\hat{p}_{0,AA}} + 2N_{Aa}\log\frac{\hat{p}_{Aa}}{\hat{p}_{0,Aa}} + 2N_{aa}\log\frac{\hat{p}_{aa}}{\hat{p}_{0,aa}}. \tag{1}$$

The full-model MLEs are given by $\hat{p}_{AA} = N_{AA}/n, \hat{p}_{Aa} = N_{Aa}/n$, *and* $\hat{p}_{aa} = N_{aa}/n$, *by Example 1.3.4 from Chapter 2. To find the sub-model MLEs, note that under* H_0, *the multinomial likelihood as a function of* θ *is*

$$\begin{aligned} \text{lik}(\theta) &= \left(\begin{array}{c} n \\ N_{AA}, N_{Aa}, N_{aa} \end{array}\right) \left(\theta^2\right)^{N_{AA}} \left(2\theta(1-\theta)\right)^{N_{Aa}} \left((1-\theta)^2\right)^{N_{aa}} \\ &= \left(\begin{array}{c} n \\ N_{AA}, N_{Aa}, N_{aa} \end{array}\right) 2^{N_{Aa}} \theta^{2N_{AA}+N_{Aa}} (1-\theta)^{N_{Aa}+2N_{aa}}. \end{aligned}$$

Maximizing the likelihood over parameters (p_{AA}, p_{Aa}, p_{aa}) *belonging to the sub-model is equivalent to maximizing the above over* θ. *Differentiating the logarithm of the above likelihood and setting it equal to 0, we obtain the MLE*

$$\hat{\theta} = \frac{2N_{AA} + N_{Aa}}{2N_{AA} + 2N_{Aa} + 2N_{aa}} = \frac{2N_{AA} + N_{Aa}}{2n}$$

for θ, *which yields the sub-model MLEs*

$$\begin{aligned}
\hat{p}_{0,AA} &= \left(\frac{2N_{AA}+N_{Aa}}{2n}\right)^2 \\
\hat{p}_{0,Aa} &= 2\left(\frac{2N_{AA}+N_{Aa}}{2n}\right)\left(\frac{N_{Aa}+2N_{aa}}{2n}\right) \\
\hat{p}_{0,aa} &= \left(\frac{N_{Aa}+2N_{aa}}{2n}\right)^2.
\end{aligned}$$

Substituting these expressions into equation (1) yields the formula for $-2\log\Lambda$ in terms of the observed counts N_{AA}, N_{Aa}, N_{aa}. The difference in dimensionality of the two models is $2-1=1$, so an approximate level-α test would reject H_0 when $-2\log\Lambda$ exceeds $\chi_1^2(\alpha)$.

Rice[10] provides an example (Example 8.5.1A) of genotype data from a population of $n = 1029$ individuals in Hong Kong, in which the alleles determine the presence of an antigen in the red blood cell. In this example, $N_{AA} = 342, N_{Aa} = 500, N_{aa} = 187$, and we may calculate $-2\log\Lambda = 0.0325$. Letting F denote the χ_1^2 CDF, the p-value of our test is $1 - F(0.0325) = 0.86$, so there is no significant evidence of deviation from the Hardy-Weinberg equilibrium.

8.3 Testing in contingency tables

8.3.1 Test of independence

We introduced the generalized likelihood ratio test, and we applied it to an example of testing the hypothesis of Hardy-Weinberg equilibrium in a population at a single diallelic locus. This was an example of testing whether the parameters of a multinomial model satisfy certain additional constraints.

Here is a second example of this type of hypothesis testing problem:

Example 8.3.1. *(Independence test). The following table (from the GSS 2008[11]) cross-classifies a random sample of 1972 people by gender and by political party identification:*

	dem	indep	repub
female	422	381	273
male	299	365	232

In this sample, approximately 39% of females identified as democrat and 25% identified as republican, while approximately 33% of males identified as democrat and 26% identified as republican. Is this significant evidence of an association between gender and party identification in the population from which this sample was drawn?

*Denote the observed counts by N_{ij} for $i = 1, 2$ and $j = 1, 2, 3$. We may model these counts as multinomial with $n = 1972$ total observations and with outcome probabilities p_{ij} for $i = 1, 2$ and $j = 1, 2, 3$. Denote by $p_{i\cdot} = \sum_j p_{ij}$ and $p_{\cdot j} = \sum_i p_{ij}$ the marginal row and column probabilities. If there is no association between gender and party identification, then $p_{ij} = p_{i\cdot}p_{\cdot j}$. Hence we wish to test the **independence null hypothesis***

$$H_0 : p_{ij} = p_{i\cdot} \cdot p_{\cdot j} \text{ for all } i = 1, 2 \text{ and } j = 1, 2, 3.$$

[10] Rice, John A. Mathematical statistics and data analysis. Cengage Learning, 2006.

[11] https://gss.norc.org/

The dimension of this sub-model may be determined as follows: The five row and column marginal probabilities $p_{1\cdot}$,$p_{2\cdot}$,$p_{\cdot 1}$, $p_{\cdot 2}$, $p_{\cdot 3}$ *specify all of the multinomial cell probabilities under* H_0. *However, they satisfy the constraints* $p_{1\cdot} + p_{2\cdot} = 1$ *and* $p_{\cdot 1} + p_{\cdot 2} + p_{\cdot 3} = 1$, *so this sub-model has dimension* $5 - 2 = 3$. *The full multinomial model has dimension 5, so the generalized likelihood ratio statistic has approximate null distribution* χ^2_2 *(since* $5 - 3 = 2$*).*

To derive the form of the likelihood ratio statistic in the above example, suppose more generally that we observe $(N_1, \ldots, N_k) \sim \text{Multinomial}\,(n, (p_1, \ldots, p_k))$, *and we wish to test the null hypothesis* $H_0 : (p_1, \ldots, p_k) \in \Omega_0$, *where* Ω_0 *represents some sub-model. The multinomial likelihood is given by*

$$\text{lik}\,(p_1, \ldots, p_k) = \left(\begin{array}{c} n \\ N_1, \ldots, N_k \end{array} \right) \prod_{i=1}^{k} p_i^{N_i}.$$

Letting $\hat{p}_{0,i}$ *denote the MLEs in this sub-model* Ω_0 *and* $\hat{p}_i$ *denote the MLEs in the full multinomial model, the generalized likelihood ratio is*

$$\Lambda = \frac{\text{lik}\,(\hat{p}_{0,1}, \ldots, \hat{p}_{0,k})}{\text{lik}\,(\hat{p}_1, \ldots, \hat{p}_k)} = \prod_{i=1}^{k} \left(\frac{\hat{p}_{0,i}}{\hat{p}_i} \right)^{N_i},$$

so

$$-2 \log \Lambda = 2 \sum_{i=1}^{k} N_i \log \frac{\hat{p}_i}{\hat{p}_{0,i}}.$$

Recall that the full model MLEs are given by $\hat{p}_i = N_i/n$, *by Example 1.3.4 of Chapter 2. Let us write* $E_i = \hat{p}_{0,i} n$, *which denotes the "expected count" for outcome* i *corresponding to the sub-model MLE* $\hat{p}_{0,i}$. *Then we obtain the simple formula*

$$-2 \log \Lambda = 2 \sum_{i=1}^{k} N_i \log \frac{N_i}{E_i}. \tag{2}$$

Example 8.3.2. *(Independence test (cont'd)). Applying equation (2) to Example 8.3.1, we must compute the sub-model MLEs. Under* H_0, *the likelihood as a function of the row and column marginal probabilities is*

$$\begin{aligned} \text{lik}\,(p_{1\cdot}, p_{2\cdot}, p_{\cdot 1}, p_{\cdot 2}, p_{\cdot 3}) &= \left(\begin{array}{c} n \\ N_{11}, \ldots, N_{23} \end{array} \right) \prod_{i=1}^{2} \prod_{j=1}^{3} (p_{i\cdot} \cdot p_{\cdot j})^{N_{ij}} \\ &= \left(\begin{array}{c} n \\ N_{11}, \ldots, N_{23} \end{array} \right) \prod_{i=1}^{2} p_i^{N_{i\cdot}} \prod_{j=1}^{3} p_{\cdot j}^{N_{\cdot j}}, \end{aligned}$$

where $N_{i\cdot} = \sum_j N_{ij}$ *and* $N_{\cdot j} = \sum_i N_{ij}$ *are the row and column marginal counts. Taking the logarithm and introducing Lagrange multipliers for the constraints, we wish to maximize*

$$\log \left(\begin{array}{c} n \\ N_{11}, \ldots, N_{23} \end{array} \right) + \sum_{i=1}^{2} N_{i\cdot} \log p_{i\cdot} + \sum_{j=1}^{3} N_{\cdot j} \log p_{\cdot j} + \lambda \left(\sum_{i=1}^{2} p_{i\cdot} - 1 \right) + \mu \left(\sum_{j=1}^{3} p_{\cdot j} - 1 \right).$$

Setting the derivatives with respect to $p_{i\cdot}$ *and* $p_{\cdot j}$ *equal to 0 yields the equations* $N_{i\cdot}/p_{i\cdot} + \lambda = 0$ *and* $N_{\cdot j}/p_{\cdot j} + \mu = 0$, *so* $p_{i\cdot} = -N_{i\cdot}/\lambda$ *and* $p_{\cdot j} = -N_{\cdot j}/\mu$. *Picking the Lagrange multipliers* $\lambda = -n$ *and*

$\mu = -n$ enforces the constraints, and we obtain the MLEs $\hat{p}_{i\cdot} = N_{i\cdot/n}/n$ and $\hat{p}_{\cdot j} = N_{\cdot j}/n$. Then the sub-model MLEs for p_{ij} are given by $\hat{p}_{0,ij} = \left(N_{i\cdot/n}/n\right)(N_{\cdot j}/n)$.

For the data of Example 8.3.1, the row and column marginal counts are given by $N_{1\cdot} = 1076$, $N_{2\cdot} = 896, N_{\cdot 1} = 721, N_{\cdot 2} = 746$, and $N_{\cdot 3} = 505$. Computing the sub-model MLEs $\hat{p}_{0,ij}$ and multiplying by n, we obtain the table of expected counts E_{ij}:

	dem	indep	repub
female	393.4	407.0	275.5
male	327.6	339.0	229.5

Applying equation (2) with the 6 observed and expected counts yields $-2\log\Lambda = 8.31$. Letting F denote the CDF of the χ^2_2 distribution, we obtain a p-value for the generalized likelihood ratio test of $1-F(8.31) = 0.016$, so there is reasonably strong evidence of an association between gender and party identification.

8.3.2 Test of homogeneity

Consider now a slightly different problem: We have independent count observations from 2 multinomial distributions, each with k outcomes: $(N_1, \ldots, N_k) \sim \text{Multinomial}\,(n, (p_1, \ldots, p_k))$ and $(M_1, \ldots, M_k) \sim \text{Multinomial}\,(m, (q_1, \ldots, q_k))$, where n and m are known sample sizes. We wish to test the **homogeneity null hypothesis**

$$H_0 : p_i = q_i \text{ for all } i = 1, \ldots, k.$$

Example 8.3.3. *(Homogeneity test). This example is from Rice*[12] *Section 13.3. When Jane Austen died, she left the novel Sandition partially completed. An admirer finished the novel, attempting to emulate Jane Austen's style. The following table counts the occurrences of six different short words in Chapters 1 and 6 of Sandition, written by Austen, and in Chapters 12 and 24 of Sandition, written by the admirer:*

	a	an	this	that	with	without
Ch. 1 and 6	101	11	15	37	28	10
Ch. 12 and 24	83	29	15	22	43	4

Is there a significant difference between the relative frequencies of these words between the two authors?

Let us model the counts from Chapters 1 and 6 as $\text{Multinomial}\,(202, (p_1, \ldots, p_6))$ and those from Chapters 12 and 24 as Multinomial $(196, (q_1, \ldots, q_6))$. Then we wish to test the homogeneity null hypothesis that $p_i = q_i$ for all $i = 1, \ldots, 6$.

To derive the generalized likelihood ratio test, note that the joint likelihood of all parameters is the product of the two multinomial likelihoods:

$$\text{lik}\,(p_1, \ldots, p_k, q_1, \ldots, q_k) = \left(\begin{array}{c} n \\ N_1, \ldots, N_k \end{array} \right) \prod_{i=1}^{k} p_i^{N_i} \times \left(\begin{array}{c} m \\ M_1, \ldots, M_k \end{array} \right) \prod_{i=1}^{k} q_i^{M_i}. \tag{3}$$

Let $\hat{p}_i$ and $\hat{q}_i$ denote the full model MLEs, and let $\hat{p}_{0,i} = \hat{q}_{0,i}$ denote the sub-model MLEs. Then the generalized likelihood ratio statistic is

[12] Rice, John A. Mathematical statistics and data analysis. Cengage Learning, 2006.

$$\Lambda = \prod_{i=1}^{k} \left(\frac{\hat{p}_{0,i}}{\hat{p}_i} \right)^{N_i} \prod_{i=1}^{k} \left(\frac{\hat{q}_{0,i}}{\hat{q}_i} \right)^{M_i},$$

so

$$-2 \log \Lambda = 2 \sum_{i=1}^{k} \left(N_i \log \frac{\hat{p}_i}{\hat{p}_{0,i}} + M_i \log \frac{\hat{q}_i}{\hat{q}_{0,i}} \right). \tag{4}$$

In the full model with two independent and unconstrained multinomial distributions, the MLEs are simply $\hat{p}_i = N_i/n$ and $\hat{q}_i = M_i/m$. Letting $E_i = \hat{p}_{0,i}n$ and $F_i = \hat{q}_{0,i}m$ denote the expected counts in the sub-model, we may write the above in the simple form

$$-2 \log \Lambda = 2 \sum_{i=1}^{k} \left(N_i \log \frac{N_i}{E_i} + M_i \log \frac{M_i}{F_i} \right).$$

To compute the above statistic, we need to compute the sub-model MLEs $\hat{p}_{0,i} = \hat{q}_{0,i}$. Under H_0, the likelihood in equation (3) simplifies to

$$\text{lik}\,(p_1, \ldots, p_k) = \left(\begin{array}{c} n \\ N_1, \ldots, N_k \end{array} \right) \left(\begin{array}{c} m \\ M_1, \ldots, M_k \end{array} \right) \prod_{i=1}^{k} p_i^{N_i + M_i}.$$

Taking the logarithm and introducing a Lagrange multiplier for the constraint $p_1 + \ldots + p_k = 1$, we wish to maximize

$$\log \left(\left(\begin{array}{c} n \\ N_1, \ldots, N_k \end{array} \right) \left(\begin{array}{c} m \\ M_1, \ldots, M_k \end{array} \right) \right) + \sum_{i=1}^{k} (N_i + M_i) \log p_i + \lambda \left(\sum_{i=1}^{k} p_i - 1 \right).$$

Setting the derivatives with respect to p_i equal to 0 , we obtain the equations $(N_i + M_i)/p_i + \lambda = 0$, so $p_i = -(N_i + M_i)/\lambda$. Choosing the Lagrange multiplier $\lambda = -(n+m)$ enforces the constraints, and we obtain the sub-model MLEs $\hat{p}_{0,i} = \hat{q}_{0,i} = (N_i + M_i)/(n+m)$.

Example 8.3.4. *(Homogeneity test (cont'd)). In the data of Example 8.3.3, we have the marginal word counts $N_1 + M_1 = 184, N_2 + M_2 = 40, N_3 + M_3 = 30, N_4 + M_4 = 59$, $N_5 + M_5 = 71$, and $N_6 + M_6 = 14$. This yields the table of expected counts*

	a	an	this	that	with	without
Ch. 1 and 6	93.4	20.3	15.2	29.9	36.0	7.1
Ch. 12 and 24	90.6	19.7	14.8	29.1	35.0	6.9

Applying equation (4) with the observed and expected counts, we obtain $-2 \log \Lambda = 19.8$. The dimensionality of the sub-model in this example is 5 (6 parameters minus 1 constraint), and the dimensionality of the full model is 10 (12 parameters minus 2 constraints), so the null distribution of $-2 \log \Lambda$ is approximately χ_5^2. Letting F denote the CDF of the χ_5^2 distribution, we obtain a p-value of $1 - F(19.8) = 0.0014$, so there is significant evidence of a difference in writing style between Austen and her admirer.

Remark 8.1.

- *In both Examples 8.3.1 and 8.3.3, we wanted to test whether there is a significant difference in the relative frequencies between the two rows. The distinction between these examples is only in the sampling design/modeling assumption: In Example 8.3.1, we treated the counts from all rows as observations from a single multinomial distribution because (we believe that) the GSS 2008 survey sampled a fixed total number of people rather than a fixed number of people of each gender. In Example 8.3.3, we modeled each row as a separate multinomial distribution with a fixed row sum.*
- *In fact, the table of expected counts, generalized likelihood ratio statistic, and degrees of freedom for the test are all the same under the two different modeling assumptions (although we derived them in two different ways), so the tests of independence and of homogeneity are procedurally the same, and the distinction between these is sometimes blurred in practice.*

Hands-on Session. (Testing gender ratios)

In a classical genetics study, Geissler (1889) studied hospital records in Saxony and compiled data on the gender ratio. The following table shows the number of male children in 6115 families having 12 children:

Number of male children	Number of families
0	7
1	45
2	181
3	478
4	829
5	1112
6	1343
7	1033
8	670
9	286
10	104
11	24
12	3

Let $X_1, \ldots, X_{6115}$ denote the number of male children in these 6115 families.

(a) Suggest two reasonable test statistics T_1 and T_2 for testing the null hypothesis

$$H_0 : X_1, \ldots, X_{6115} \overset{IID}{\sim} \text{Binomial}(12, 0.5).$$

(This is intentionally open-ended; try to pick T_1 and T_2 to "target" different possible alternatives to the above null.) Compute the values of T_1 and T_2 for the above data.

(b) Perform a simulation to simulate the null distributions of T_1 and T_2. (For example: Simulate 6115 independent samples $X_1, \ldots, X_{6115}$ from Binomial$(12, 0.5)$, and compute T_1 on this sample. Do this 1000 times to obtain 1000 simulated values of T_1. Do the same for T_2.) Plot the histograms of the simulated null distributions of T_1 and T_2. Using your simulated values, compute approximate p-values of the hypothesis tests based on T_1 and T_2 for the above data. For either of your tests, can you reject H_0 at significance level $\alpha = 0.05$?

(c) In this example, why might the null hypothesis H_0 not hold? (Please answer this question regardless of your findings in part (b).)

Solution.

(a) There are many possible answers. We may take T_1 to be the average number of male children per family,

$$T_1 = \bar{X},$$

and perform a two-sided test based on T_1 to check whether roughly half of the children are male. We may take T_2 to be Pearson's chi-squared statistic

$$T_2 = \sum_{k=0}^{12} (O_k - E_k)^2 / E_k$$

where O_k is the number of families with k male children and E_k is the expected number under the hypothesized binomial distribution, i.e. $E_k = 6115 \times \left(\begin{array}{c} 12 \\ k \end{array} \right) (0.5)^{12}$, and perform a one-sided test that rejects for large T_2 to check whether the shape of the observed distribution of $X_1, \ldots, X_{6115}$ matches the shape of the binomial PDF.

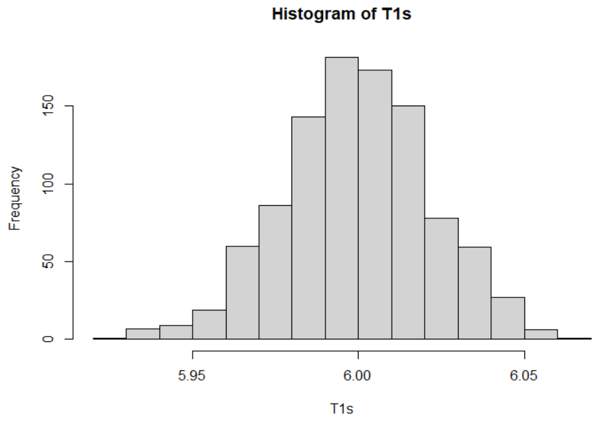
(b) $\mathbb{R}$ code corresponding to the above T_1 and T_2 is as follows:

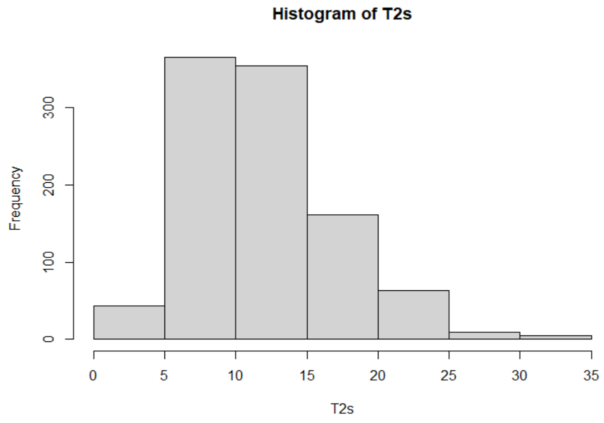
```
ks = seq(0,12)
counts = c(7,45,181,478,829,1112,1343,1033,670,286,104,24,3)
expected = 6115*choose(12,ks)*(0.5^12)

T1_obs = sum(ks*counts)/6115
T2_obs = sum((counts-expected)^2/expected)

T1s = numeric(1000)
T2s = numeric(1000)
for (i in 1:1000) {
  X = rbinom(6115, 12, 0.5)
  T1s[i] = mean(X)
  counts = numeric(13)
  for (k in 0:12) {
    counts[k+1] = length(which(X==k))
    }
  T2s[i] = sum((counts-expected)^2/expected)
  }
hist(T1s)
hist(T2s)
T1_pvalue = length(which(T1s<T1_obs))/1000 * 2
T2_pvalue = length(which(T2s>T2_obs))/1000
```

Histograms of the null distributions of T_1 and T_2 are below:





The values of the test statistics for the observed data are $T_1 = 5.77$ and $T_2 = 249$, which are both far outside the range of the simulated null distributions above. The simulated p-values for the two tests are both < 0.001, and there is strong evidence that H_0 is not correct.

(c) There may be both biological and sociological reasons why H_0 is false. Biologically, the human male-to-female sex ratio at birth is not exactly $1 : 1$. The probability p that a child is male might also vary from family to family. The sexes of children within a family might be dependent; in particular, one source of dependence is the presence of identical twins.
Sociologically, there may be a relationship between family size and the sex ratio of children in the family because the current sex ratio influences parents' decision of whether to have another child. Note that the given data is only for families with 12 children, which is quite large even for that time. There is a noticeable bias towards families with more girls than boys, which may be explained if parents tended to continue having children when their current children were predominantly female.

Hands-on Session. (Power Comparisons)

Consider the problem of testing

$$H_0 : X_1, \dots, X_n \overset{\text{IID}}{\sim} \mathcal{N}(0,1)$$
$$H_1 : X_1, \dots, X_n \overset{\text{IID}}{\sim} \mathcal{N}(\mu,1)$$

at significance level $\alpha = 0.05$, where $\mu > 0$. We've seen four tests that may be applied to this problem, summarized below:

- Likelihood ratio test: Reject H_0 when $\bar{X} > \frac{1}{\sqrt{n}} Z_{0.05}$.
- t-test: Reject H_0 when $T := \sqrt{n}\bar{X}/S > t_{n-1;0.05}$, where $S^2 = \frac{1}{n-1} \sum_i \left(X_i - \bar{X}\right)^2$.
- Wilcoxon signed rank test: Reject H_0 when $W_+ > \frac{n(n+1)}{4} + \sqrt{\frac{n(n+1)(2n+1)}{24}} Z_{0.05}$, where W_+ is the Wilcoxon signed rank statistic.
- Sign test (from Problem 4 of TD-3): Reject H_0 when $S > \frac{n}{2} + \sqrt{\frac{n}{4}} Z_{0.05}$, where S is the number of positive values in $X_1, \dots, X_n$.

(For the Wilcoxon and sign test statistics, we are using the normal approximations for their null distributions.) These tests are successively more robust to violations of the $\mathcal{N}(0,1)$ distributional assumption imposed by H_0.

(a) For $n = 100$, verify numerically that these tests have significance level close to α, in the following way: Perform 10,000 simulations. In each simulation, draw a sample of 100 observations from $\mathcal{N}(0, 1)$, compute the above four test statistics $\bar{X}, T, W_+$, and S on this sample, and record whether each test accepts or rejects H_0. Report the fraction of simulations for which each test rejected H_0, and confirm that these fractions are close to 0.05.

(b) For $n = 100$, numerically compute the powers of these tests against the alternative H_1, for the values $\mu = 0.1, 0.2, 0.3$, and 0.4. Do this by performing 10,000 simulations as in part (a), except now drawing each sample of 100 observations from $\mathcal{N}(\mu, 1)$ instead of $\mathcal{N}(0, 1)$. (You should be able to re-use most of your code from part (a).) Report your computed powers either in a table or visually using a graph.

(c) How do the powers of the four tests compare, when testing against a normal alternative? Your friend says, "We should always use the testing procedure that makes the fewest distributional assumptions, because we never know in practice, for example, whether the variance is truly 1 or whether data is truly normal." Comment on this statement. Rice says, "It has been shown that even when the assumption of normality holds, the [Wilcoxon] signed rank test is nearly as powerful as the t test. The [signed rank test] is thus generally preferable, especially for small sample sizes." Do your simulated results support this conclusion?

Solution.

(a) The code below runs the simulations for the null case ($\mu = 0$) as well as for $\mu = 0.1, 0.2, 0.3, 0.4$:

```
set.seed(1)
n = 100
B = 10000
for (mu in c(0,0.1,0.2,0.3,0.4)) {
  output.Z = numeric(B)
  output.T = numeric(B)
  output.W = numeric(B)
  output.S = numeric(B)
  for (i in 1:B) {
    X = rnorm(n, mean=mu, sd=1)
    if (mean(X) > 1/sqrt(n)*qnorm(0.95)) {
      output.Z[i] = 1
    } else {
        output.Z[i] = 0
    }
    T = t.test(X)$statistic
    if (T > qt(0.95,df=n-1)) {
      output.T[i] = 1
    } else {
        output.T[i] = 0
    }
    W = wilcox.test(X)$statistic
    if (W > n*(n+1)/4+sqrt(n*(n+1)*(2*n+1)/24)*qnorm(0.95)) {
      output.W[i] = 1
    } else {
        output.W[i] = 0
    }
    S = length(which(X>0))
    if (S > n/2+sqrt(n/4)*qnorm(0.95)) {
      output.S[i] = 1
    } else {
        output.S[i] = 0
    }
  }
  print(paste("mu = ", mu))
  print(paste("Z: ", mean(output.Z)))
  print(paste("T: ", mean(output.T)))
  print(paste("W: ", mean(output.W)))
  print(paste("S: ", mean(output.S)))
}
```

Under H_0 (case $\mu = 0$), we obtained the results:

Test stat	Type-I Error
Likelihood ratio test	0.0507
t-test	0.0505
Wilcoxon signed rank test	0.053
Sign test	0.0441

(b) Under these alternatives, we obtained the results:

Test stat	Power over $\mathcal{N}(\mu, 1)$			
	$\mu = 0.1$	$\mu = 0.2$	$\mu = 0.3$	$\mu = 0.4$
Likelihood ratio test	0.2631	0.6356	0.913	0.9895
t-test	0.261	0.6306	0.9085	0.9885
Wilcoxon signed-rank test	0.252	0.6164	0.8978	0.9847
Sign test	0.1805	0.4617	0.7521	0.9318

(c) The powers of the tests against $\mathcal{N}(\mu, 1)$ decrease as we increasingly relax the distributional assumptions (from $\mathcal{N}(0, 1)$ to $\mathcal{N}(0, \sigma^2)$ to any symmetric PDF f about 0 to any PDF f with median 0). The sign test makes the fewest distributional assumptions under H_0, but its power is substantially lower than the other three tests. Hence if we have good reason to believe that the data distribution under H_0 is symmetric (for example, if each data value is the difference of paired samples (X_i, Y_i), and (X_i, Y_i) should have the same distribution as (Y_i, X_i) under H_0), then we should at least opt for using the Wilcoxon test. The difference in powers between the Wilcoxon test, t-test, and the most-powerful likelihood ratio test is indeed very small, which supports Rice's claim (at least for the tested sample size $n = 100$).

9 Experimental design

9.1 Steps of a statistical study

A "typical" statistical study might consist of the following steps:

1. Identify/formulate the question of interest
2. Design an experiment or study to collect data that addresses this question
3. Clean, visualize, and explore the data
4. Draw an inference from the data to answer the original question

So far, our focus has been on Step 4. (We discussed briefly ideas such as hanging histogram plots and QQ plots for Step 3.) Now we'll discuss some aspects of Step 2 in the context of two-sample hypothesis testing. We try to address the following questions:

- How can we eliminate or minimize the influence of confounding factors?[13]
- How can we reason about the size of the study needed to identify an effect of interest?
- How can we design the experiment so as to maximize the chance of identifying this effect?

9.2 Case study: Peer grading students in statistics course

- **Context**: Grading student homework assignments in large classes is time-consuming and costly, perhaps prohibitively so in Massive Open Online Courses (MOOCs) with thousands or tens of thousands of students.
- **Possible solution**: Have students grade each other (peer grading).
- **Question of interest**: Can peer grading actually increase student learning?

Justice Anthony Kennedy, in Supreme Court case *Owasso v. Valvo*: "Correcting a classmate's work can be as much a part of the assignment as taking the test itself. It is a way to teach material again in a new context, and it helps show students how to assist and respect fellow pupils."

9.2.1 A simple design

Suppose there are 300 students undertaking a statistics course. Divide them into two groups, "peer-grading" and "control". Have only the students in the peer-grading group grade their peers and compare learning (e.g., test scores) between the two groups at the end of the quarter.

Problem: Student performance is influenced by many confounding factors – their class year, previous coursework and knowledge of statistics, etc.

Simple solution: Randomly assign students to the two groups so that confounding factors tend to be balanced between the groups. For this design, we might use a two-sample t-test:

[13] see examples in Rice, John A. Mathematical statistics and data analysis. Cengage Learning, 2006., Section 11.4.

Let $X_1, \ldots, X_n$ be final exam scores of the peer-grading group, $Y_1, \ldots, Y_m$ those of the control group. Supposing that $X_1, \ldots, X_n \sim \mathcal{N}\left(\mu_X, \sigma^2\right), Y_1, \ldots, Y_m \sim \mathcal{N}\left(\mu_Y, \sigma^2\right)$, test

$$\begin{aligned} H_0 &: \mu_X = \mu_Y \\ H_1 &: \mu_X > \mu_Y \end{aligned}$$

using the two-sample T-statistic $T = \frac{\bar{X}-\bar{Y}}{S_p\sqrt{\frac{1}{n}+\frac{1}{m}}}$, where S_p^2 is the pooled sample variance discussed in Section 4.1.

What is the chance that we identify a significant effect (reject H_0)?

Calculating the power

Here $n+m = 300$ is fairly large, so we expect S_p^2 to be a very accurate estimate of σ^2. Let's assume for simplicity that we know σ^2, and perform the test using

$$Z = \frac{\bar{X}-\bar{Y}}{\sigma\sqrt{\frac{1}{n}+\frac{1}{m}}}.$$

Recall,

$$\bar{X}-\bar{Y} \sim \mathcal{N}\left(\mu_X-\mu_Y, \sigma^2\left(\frac{1}{n}+\frac{1}{m}\right)\right).$$

Under H_0, $Z \sim \mathcal{N}(0,1)$, so a one-sided test rejects when $Z > z(\alpha)$.

Under H_1, $Z \sim \mathcal{N}(d,1)$, where

$$d = \frac{\mu_X-\mu_Y}{\sigma\sqrt{\frac{1}{n}+\frac{1}{m}}}.$$

The power of the test increases with d:

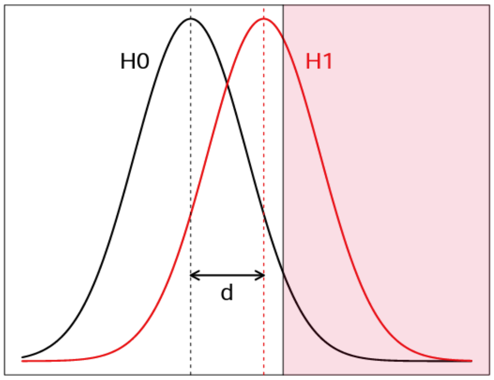


Figure 3: Distributions of Z under H_0 and H_1.

The separation

$$d = \frac{\mu_X-\mu_Y}{\sigma}\sqrt{\frac{1}{\frac{1}{n}+\frac{1}{m}}}$$

is determined by:

- The real difference in mean test scores $\mu_X - \mu_Y$.
- The standard deviation of test scores (i.e., "noise" level) σ.
- The sample sizes n and m.

The quantity $\frac{\mu_X - \mu_Y}{\sigma}$ is called the **effect size** – it measures the size of the mean difference in terms of the number of standard deviations of the noise.

The power of the test is

$$\mathbb{P}_{H_1}[Z > z(\alpha)] = \mathbb{P}_{H_1}[Z - d > z(\alpha) - d] = 1 - \Phi(z(\alpha) - d),$$

where $\Phi(x)$ is the standard normal CDF, and we used the fact that $Z - d \sim \mathcal{N}(0, 1)$ under H_1.

Subject to the constraint of $n + m = 300$ total students, d is maximized when we choose $n = m = 150$ students per group. The effect size identified by the study (in retrospect) was 0.11. So

$$d = \frac{\mu_X - \mu_Y}{\sigma}\sqrt{\frac{n}{2}} = 0.95.$$

At level $\alpha = 0.05$, the above power is only 0.244! In other words, had we done this experiment, we would have only had a 24% chance of rejecting H_0 at level $\alpha = 0.05$.

Typical p-value

We can also think in terms of the p-value we would have obtained. If the test statistic we observed were Z, then the p-value would be the upper tail probability

$$P = 1 - \Phi(Z).$$

(P and Z here are both random, depending on the outcome of the experiment)

Under H_1, $Z \sim \mathcal{N}(d, 1)$, so the median value of Z is d. Since $x \mapsto 1 - \Phi(x)$ is monotone (decreasing), the median value of P is $1 - \Phi(d)$. So a "typical" p-value from this experiment would have been $1 - \Phi(d)$. For $d = 0.95$, this p-value is 0.17.

Both of these calculations indicate that the study would be **underpowered** – the effect size is too small to be detected with statistical significance if the sample size is 300 students.

How many samples are needed?

Suppose we would like the power to be much larger, say 0.9, under a level $\alpha = 0.05$ test. How many students would we need? If we have n students in each of the peer-grading and control groups, set

$$0.9 = 1 - \Phi(z(\alpha) - d) = 1 - \Phi\left(z(0.05) - 0.11\sqrt{\frac{n}{2}}\right)$$

and solve for n:

$$\begin{aligned}\Phi\left(z(0.05) - 0.11\sqrt{\frac{n}{2}}\right) &= 0.1\\ \Rightarrow z(0.05) - 0.11\sqrt{\frac{n}{2}} &= \Phi^{-1}(0.1) = -z(0.1)\\ \Rightarrow n &= 2\left(\frac{z(0.05)+z_{0.1}}{0.11}\right)^2 \approx 1416\end{aligned}$$

We would need $2n \approx 2832$ total students. This amounts to doing this experiment for $5-10$ years of students from the statistics course.

Remark 9.1. ***Effect sizes in education***

The previous calculations assumed we knew the effect size was 0.11. In reality, we don't know this ahead of time. However, we can compare to what we know:

- *Classroom discussion – 0.82*
- *Computer-assisted instruction – 0.45*
- *Teacher education – 0.12*
- *Charter schools – 0.07*

These numbers are from the 2015 Hattie ranking[14]*, which lists effect sizes for 195 different educational influences/approaches, determined from aggregating previous experimental studies. In education, an effect size larger than* 0.4 *is typically considered strong.*

9.2.2 A different design to improve power

Main problem: There is too much variation in student performance, compared to the size of the improvement from peer-grading.

Idea: Compare each student to himself/herself.

Implementation: Divide statistics course students into 2 units [15], with a quiz at the end of each unit. Assign each student to do peer-grading for one unit, and no peer-grading for the other unit. (To handle the possible confounding factor that one exam is easier than the other, randomly choose which unit each student does peer-grading.) In other words, set up an experiment with paired samples rather than two independent samples.

Calculating the power for paired samples: Why does this help (and how much does it help by)?

Suppose there are n students. Let $X_1, \ldots, X_n$ be their quiz scores in the peer-grading unit, and $Y_1, \ldots, Y_n$ their scores in the control unit. For this design, we might use a one-sample (a.k.a. paired two-sample) t-test:
Let $D_i = X_i - Y_i$, and reject H_0 for large values of the t-statistic

$$T = \frac{\sqrt{n}\bar{D}}{S}.$$

[14] https://visible-learning.org/hattie-ranking-influences-effect-sizes-learning-achievement/

[15] The real study used 4 units instead of 2.

Here, $\bar{D}$ and S^2 are the sample mean and variance of the D_i's.

Assume $X_i \sim \mathcal{N}(\mu_X, \sigma^2)$ and $Y_i \sim \mathcal{N}(\mu_Y, \sigma^2)$, as before. Since X_i and Y_i correspond to the same student, they are likely very correlated. Let's suppose (X_i, Y_i) is bivariate normal with correlation ρ:

$$(X_i, Y_i) \sim \mathcal{N}\left(\left(\begin{array}{c} \mu_X \\ \mu_Y \end{array}\right), \left(\begin{array}{cc} \sigma^2 & \rho\sigma^2 \\ \rho\sigma^2 & \sigma^2 \end{array}\right)\right).$$

Then $D_i = X_i - Y_i$ is normally distributed, with mean $\mathbb{E}[D_i] = \mu_X - \mu_Y$ and variance

$$\begin{aligned}
\operatorname{Var}[D_i] &= \operatorname{Cov}[X_i - Y_i, X_i - Y_i] \\
&= \operatorname{Cov}[X_i, X_i] - \operatorname{Cov}[X_i, Y_i] - \operatorname{Cov}[Y_i, X_i] + \operatorname{Cov}[Y_i, Y_i] \\
&= \sigma^2 - \rho\sigma^2 - \rho\sigma^2 + \sigma^2 \\
&= 2\sigma^2(1-\rho).
\end{aligned}$$

Since n is large, S^2 should be very close to $\operatorname{Var}[D_i] = 2\sigma^2(1-\rho)$. Let's suppose again for simplicity that we know $2\sigma^2(1-\rho)$, and consider the test statistic

$$Z = \frac{\sqrt{n}\bar{D}}{\sqrt{2\sigma^2(1-\rho)}}.$$

We have $\bar{D} \sim \mathcal{N}\left(\mu_X - \mu_Y, \frac{2\sigma^2(1-\rho)}{n}\right)$.

Under H_0, $Z \sim \mathcal{N}(0,1)$, so a level-α test rejects when $Z > z(\alpha)$.

Under H_1, $Z \sim \mathcal{N}(d,1)$, where

$$d = \frac{\mu_X - \mu_Y}{\sigma}\sqrt{\frac{n}{2(1-\rho)}}.$$

Compared to having two independent samples of size n (one peer-grading, one control), we gain a factor of $1/\sqrt{1-\rho}$ in d. You can think of this as either reducing the effective variance from σ^2 (in the case of unpaired samples) to $\sigma^2(1-\rho)$ (in the case of paired samples), or as increasing the effective sample size from n (in the case of unpaired samples) to $n/(1-\rho)$ (in the case of paired samples). The factor $1-\rho$ is called the **relative efficiency** of the unpaired design to the paired design.

Example 9.2.1. *If $\rho = 0.9$, then the relative efficiency is 0.1, and a paired design with n pairs yields the same power as an unpaired design with two independent samples of size $10n$.*

Examples of paired designs

- Before-and-after studies on the same subjects
- Twin studies
- Subject matching by covariates (e.g., in a medical study, matching by age, weight, severity of condition, etc.)

Matching by covariates was also used in the statistics students experiment: Rather than randomly choosing, for each student, which unit they did peer-grading, each student was paired with the "most similar" other student based on gender, race, previous statistics background, class year, etc. using a matching algorithm. One student in each pair was then randomly assigned to peer grade in unit 1, and the other to peer grade in unit 2. Pairing by covariates is a special case of a **randomized block design**, which groups subjects into blocks having similar characteristics.

Summary of the study

- The estimated (short-term) effect size was 0.11. Despite the small size of the effect, it was found to be statistically significant with p-value 0.002.
- Long-term effect was assessed by comparing performance on the questions corresponding to each unit in the final exam; the estimated effect size was 0.12, with p-value 0.001.

Conclusion: Peer grading yielded a small but real improvement in student learning[16].

9.3 Addendum: S^2 is close to σ^2 for large n

As $n \to \infty$, the sample variance $S^2 \to \sigma^2$ in probability. Why?

Suppose $X_1, \ldots, X_n$ are IID with mean 0 and variance σ^2.

$$\begin{aligned} S^2 &= \frac{1}{n-1} \sum_{i=1}^{n} \left(X_i - \bar{X}\right)^2 \\ &= \frac{1}{n-1} \left(\sum_{i=1}^{n} X_i^2 - n\bar{X}^2 \right) \\ &= \frac{n}{n-1} \cdot \frac{1}{n} \sum_{i=1}^{n} X_i^2 - \frac{n}{n-1} \bar{X}^2. \end{aligned}$$

As $n \to \infty$, $\frac{n}{n-1} \to 1$. Also by the LLN, $\frac{1}{n}\sum_{i=1}^{n} X_i^2 \to \sigma^2$ and $\bar{X} \to 0$ in probability.

The functions $(x, y) \mapsto x - y$ and $(x, y) \mapsto xy$ are continuous. So if $X_n \to a$ and $Y_n \to b$ in probability, then the Continuous Mapping Theorem implies $X_n - Y_n \to a - b$ and $X_n Y_n \to ab$ in probability. Then

$$S^2 = \frac{n}{n-1} \cdot \frac{1}{n} \sum_{i=1}^{n} X_i^2 - \frac{n}{n-1} \bar{X}^2 \to 1 \cdot \sigma^2 - 1 \cdot 0 \cdot 0 = \sigma^2$$

in probability. Clearly this also holds if $X_1, \ldots, X_n$ are IID with mean μ and variance σ^2, because S^2 doesn't depend on μ. Note that we didn't assume that the X_i's are normally distributed – this argument holds as long as $X_1, \ldots, X_n$ are IID with finite variance.

10 Testing multiple hypotheses

10.1 The multiple testing problem: More p-values, more problems

XKCD by Randall Monroe[17] takes esoteric math and science concepts and turns them into jokes. In one example, Monroe tackles the issue of multiple hypothesis testing: If you test many hypotheses simultaneously without adjusting your significance cutoff (e.g., $p < 0.05$), false positives are going to happen

[16] Details available at: Sun, D. L., Harris, N., Walther, G., & Baiocchi, M. (2015). "Peer assessment enhances student learning: The results of a matched randomized crossover experiment in a college statistics class." PloS one.

[17] https://xkcd.com/882/

more than you might expect.

Motivating example: In the related edition of XKCD, two characters want to know if jelly beans cause acne. Scientists investigate this claim and find no link between jelly beans and acne. That is, the scientists test the null hypothesis, "There is no statistically significant relationship between jelly bean consumption and acne." The results will not surprise you.

Objective of the case study: In this joke example, the scientists test one hypothesis, calculating one p-value and comparing that one p-value to a critical value (here 0.05). No problem so far. But, what if I am concerned that one specific color out of a possible, say, 20 jelly bean colors cause acne?

Findings of the case study: So green jelly beans cause acne? We can see the headlines now. Notice that in part of this joke front-page news there is a comment "only 5% chance of coincidence." Is that right? If the scientists had tested a single hypothesis, then yes. However, that's not what happened. The scientists tested 20 hypotheses. So what are the odds this result happened by chance?
Thoughts on the case study: p-value tells you the likelihood of getting a result as extreme or more extreme by chance. That means for a single hypothesis test, the p-value tells you the likelihood of getting something like your result by chance. What about if we tested 20 independent hypotheses with a cutoff of 0.05? In that case,

$$P(\text{at least one false positive}) = 1 - P(\text{no results are significant}) = 1 - (1 - 0.05)^{20} \approx 0.64.$$

There is a 64% chance of at least one false positive. Said another way, it is more likely than not that this experiment will yield at least one false positive just by chance. What is the possible remedy?

The simplest approach is to divide your cut-off value by the number of simultaneous hypotheses. This process is called a Bonferroni correction In this case, that would be

$$\text{Cutoff } = \frac{0.05}{20} = 0.0025$$

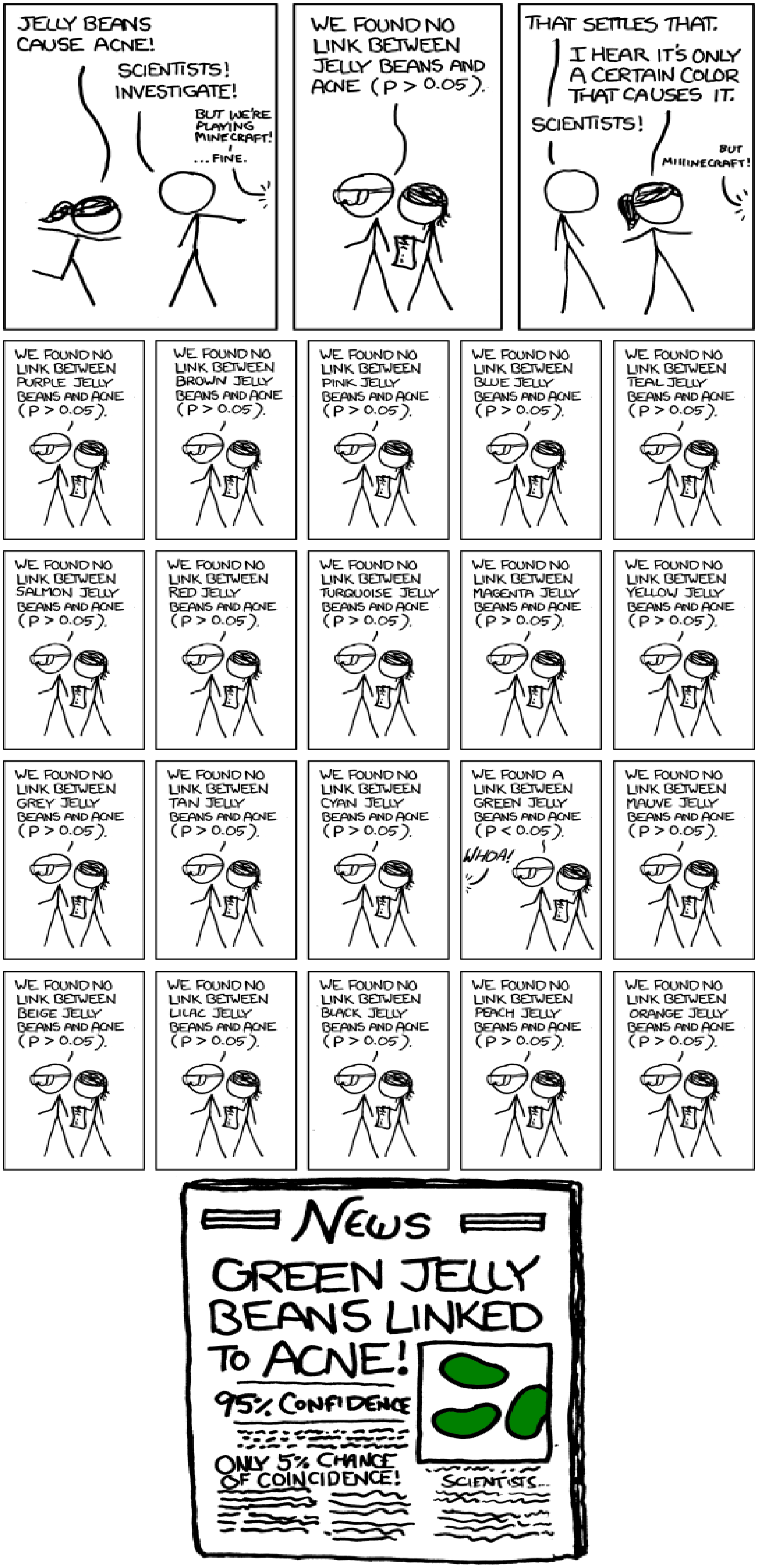
You may think, "That's a very strict cutoff." You're right. This cutoff will do a great job of preventing false positives. In fact, we can prove it.

$$P(\text{ at least one false positive }) = 1 - P(\text{ no results are significant }) = 1 - (1 - 0.0025)^{20} \approx 0.0488.$$

This number, 0.0488, can be thought of as the cut-off equivalent. If we were to somehow condense all 20 tests into 1, the cutoff for this test would be 0.488. However, as you might expect, this process results in more false negatives than would be expected from a single hypothesis test. In fact, you can prove that the false negative rate tends toward 1 as the number of tests increases[18]. That is, if you do a lot of simultaneous tests with this method, you'll fail to reject the null hypothesis nearly every time, regardless of whether there is actually a relationship in our data.

The Bonferoni correction is still useful, though. If having even one false positive would mean disaster for your work, then the Bonferoni correction may be the way to go, as it is quite conservative. Likewise, if you are testing only a small number of hypotheses (say < 25) and we expect (based on prior knowledge) that only one or two are true, the Bonferoni correction may be the way to go. One option is to control

[18] Efron, B. (2004). Large-scale simultaneous hypothesis testing: the choice of a null hypothesis. Journal of the American Statistical Association, 99(465), 96-104.


JELLY BEANS CAUSE ACNE!
SCIENTISTS! INVESTIGATE!
BUT WE'RE PLAYING MINECRAFT!
... FINE.
WE FOUND NO LINK BETWEEN JELLY BEANS AND ACNE (P > 0.05).
THAT SETTLES THAT.
I HEAR IT'S ONLY A CERTAIN COLOR THAT CAUSES IT.
SCIENTISTS!
BUT MIIIINECRAFT!
WE FOUND NO LINK BETWEEN PURPLE JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN BROWN JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN PINK JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN BLUE JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN TEAL JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN SALMON JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN RED JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN TURQUOISE JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN MAGENTA JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN YELLOW JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN GREY JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN TAN JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN CYAN JELLY BEANS AND ACNE (P > 0.05).
WE FOUND A LINK BETWEEN GREEN JELLY BEANS AND ACNE (P < 0.05).
WHOA!
WE FOUND NO LINK BETWEEN MAUVE JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN BEIGE JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN LILAC JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN BLACK JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN PEACH JELLY BEANS AND ACNE (P > 0.05).
WE FOUND NO LINK BETWEEN ORANGE JELLY BEANS AND ACNE (P > 0.05).
NEWS
GREEN JELLY BEANS LINKED TO ACNE!
95% CONFIDENCE
ONLY 5% CHANCE OF COINCIDENCE!
SCIENTISTS...

the False Discovery Rate (FDR).[19] For a theoretical description, see Benjamini and Hochberg (1995).[20]

Multiple testing problem: If we test n true null hypotheses at level α, then on average we'll still (falsely) reject αn of them. Examples are as follows:

- Test the safety of a drug in terms of a dozen different side effects.
- Test whether a disease is related to 10,000 different gene expressions.

What are some ways we can think about acceptance/rejection errors across multiple hypothesis tests/experiments? What statistical procedures can control these measures of errors?

10.2 The Bonferroni correction

Consider testing n different null hypotheses $H_0^{(1)}, \dots, H_0^{(n)}$, all of which are, in fact, true. One goal we might set is to ensure

$$\mathbb{P}\left[\text{reject any null hypothesis}\right] \leq \alpha.$$

A simple and commonly-used method of achieving this is called the **Bonferroni** method: Perform each test at significance level α/n, instead of level α. Verification:

$$\begin{aligned}
\mathbb{P}\left[\text{Reject any null hypothesis}\right] &= \mathbb{P}\left[\left\{ \text{ Reject } H_0^{(1)} \right\} \cup \ldots \cup \left\{ \text{ Reject } H_0^{(n)} \right\}\right] \\
&\leq \mathbb{P}\left[\text{ Reject } H_0^{(1)} \right] + \ldots + \mathbb{P}\left[\text{ Reject } H_0^{(n)} \right] \\
&= \frac{\alpha}{n} + \ldots + \frac{\alpha}{n} = \alpha
\end{aligned}$$

10.3 Family-wise error rate

More generally, suppose we test n null hypotheses, n_0 of which are true and $n - n_0$ of which are false. The results of the tests might be tabulated as follows:

	H_0 is true	H_0 is false	Total
Reject H_0	V	S	R
Accept H_0	U	T	$n - R$
Total	n_0	$n - n_0$	n

$R = \#$ rejected null hypotheses,
$V = \#$ type I errors,
$T = \#$ type II errors.

Remark 10.1. *We consider n_0 and $n - n_0$ to be fixed quantities. The number of hypotheses we reject, R, as well as the cell counts U, V, S, T, are random, as they depend on the data observed in each experiment.*

[19] https://www.biostathandbook.com/multiplecomparisons.html

[20] Benjamini, Y., & Hochberg, Y. (1995). Controlling the false discovery rate: a practical and powerful approach to multiple testing. Journal of the Royal Statistical Society: Series B (Methodological), 57(1), 289-300.

The **family-wise error rate** (FWER) is the probability of falsely rejecting at least one true null hypothesis,

$$\mathbb{P}[V \geq 1].$$

A procedure controls FWER at level α if $\mathbb{P}[V \geq 1] \leq \alpha$, regardless of the (possibly unknown) number of true null hypotheses n_0.

Bonferroni controls FWER: Without loss of generality, let $H_0^{(1)}, \ldots, H_0^{(n_0)}$ be the true null hypotheses.

$$\begin{aligned}\mathbb{P}[V \geq 1] &= \mathbb{P}\left[\left\{\text{ Reject } H_0^{(1)}\right\} \cup \ldots \cup \left\{\text{ Reject } H_0^{(n_0)}\right\}\right] \\ &\leq \mathbb{P}\left[\text{ Reject } H_0^{(1)}\right] + \ldots + \mathbb{P}\left[\text{ Reject } H_0^{(n_0)}\right] \\ &= \frac{\alpha}{n} + \ldots + \frac{\alpha}{n} = \frac{\alpha n_0}{n} \leq \alpha.\end{aligned}$$

10.4 Thinking in terms of p-values

Many multiple-testing procedures are formulated as operating on the p-values returned by individual tests, rather than on the original data or the test statistics that were used.

For example, Bonferroni may be described as follows: Reject those null hypotheses whose corresponding p-values are at most α/n.

Key advantages:

- Abstracts away details about how individual tests were performed.
- Applicable regardless of which tests/test statistics were used for each experiment.
- Allows for meta-analyses of previous experiments without access to the original data.

10.5 The null distribution of a p-value

Suppose a null hypothesis H_0 is true, and we perform a statistical test of H_0 and obtain a p-value P. What is the distribution of P?

If our test statistic T has a continuous distribution under H_0 with CDF F, and we reject for small values of T, then the p-value is just the lower tail probability

$$P = F(T).$$

For any $t \in (0, 1)$

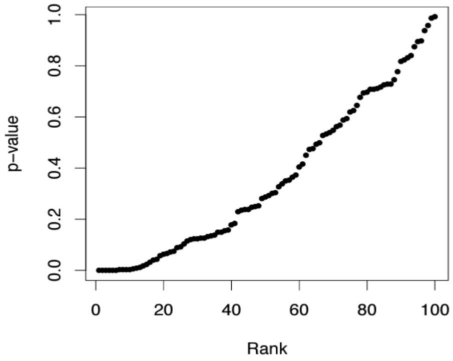
$$\mathbb{P}[P \leq t] = \mathbb{P}[F(T) \leq t] = \mathbb{P}\left[T \leq F^{-1}(t)\right] = F\left(F^{-1}(t)\right) = t.$$

So $P \sim \text{Uniform}(0,1)$. Similarly, $P \sim \text{Uniform}(0,1)$ if we reject for large T, or both large and small T.[21]

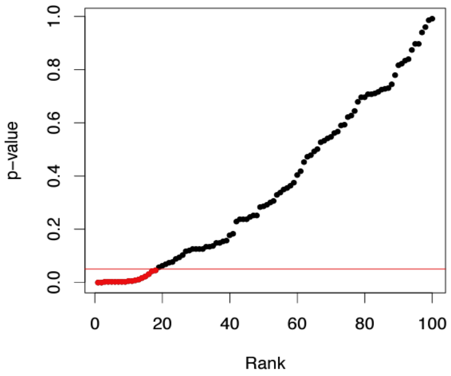
[21] If T has a discrete distribution under H_0, then so does P, so the null distribution of P wouldn't be exactly Uniform $(0, 1)$.

10.6 Ordered p-value plots

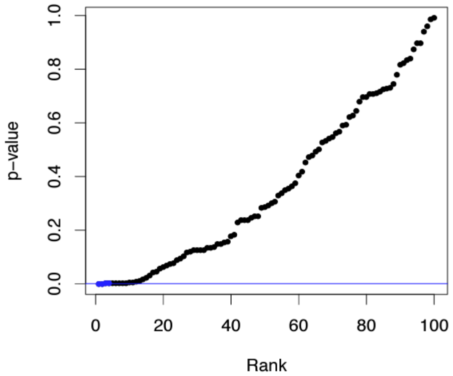
We can understand multiple testing procedures visually in terms of the plot of the ordered p-values (sorted from smallest to largest):



Applying each test at level 0.05, we reject the null hypotheses corresponding to the below 18 red points.



Applying the Bonferroni correction, we reject null hypotheses with p-value less than 0.0005, corresponding to the below 4 blue points.



10.7 False discovery rate

	H_0 is true	H_0 is false	Total
Reject H_0	V	S	R
Accept H_0	U	T	$n-R$
Total	n_0	$n-n_0$	n

Controlling the FWER $\mathbb{P}[V \geq 1]$ may be too conservative and greatly reduce our power to detect real effects, especially when n (the total number of tested hypotheses) is large.

In many modern "large-scale testing" applications, the focus has shifted to the **false-discovery proportion** (FDP)

$$\text{FDP} = \begin{cases} \frac{V}{R} & R \geq 1 \\ 0 & R = 0, \end{cases}$$

and on procedures that control its expected value $\mathbb{E}[\text{FDP}]$, called the **false-discovery rate** (FDR).

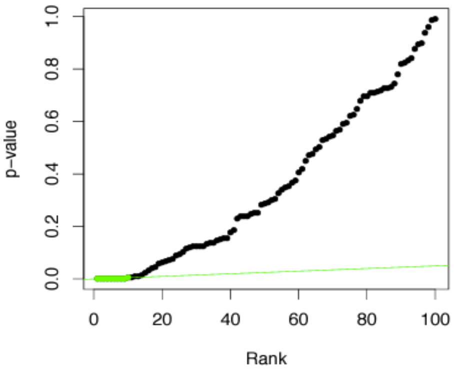
10.8 FWER vs. FDR

Controlling FDR is a shift in paradigm – we are willing to tolerate some type I errors (false discoveries), as long as most of the discoveries we make are still true.

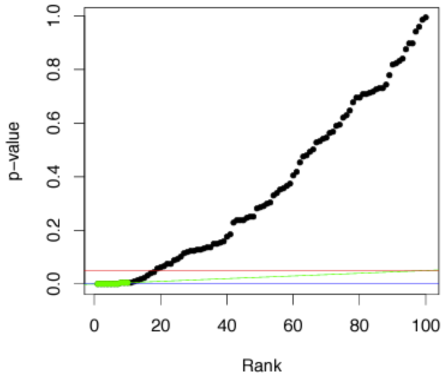
It has been argued that in applications where the statistical test is thought of as providing a "definitive answer" for whether an effect is real, FWER control is still the correct objective. In contrast, for applications where the statistical test identifies candidate effects that are likely to be real and which merit further study, it may be better to target FDR control.

10.9 The Benjamini-Hochberg procedure

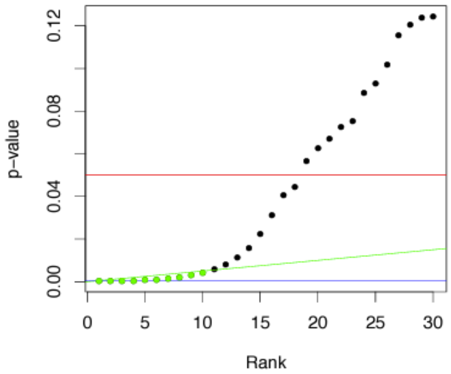
The **Benjamini-Hochberg (BH)** procedure compares the sorted p-values to a diagonal cutoff line, finds the largest p-value that still falls below this line, and rejects the null hypotheses for the p-values up to and including this one.



To control FDR at level q, the diagonal cutoff line is set to equal the Bonferroni level q/n at the smallest p-value and to equal the uncorrected level q at the largest p-value.



Here's the same picture, zoomed in to the 30 smallest p-values. In this example, the BH procedure rejects the 10 null hypotheses corresponding to the points in green.



Formally, the BH procedure at level q is defined as follows:

1. Sort the p-values. Call them $P_{(1)} \leq \ldots \leq P_{(n)}$.
2. Find the largest r such that $P_{(r)} \leq \frac{qr}{n}$.
3. Reject the null hypotheses $H_{(1)}, \ldots, H_{(r)}$.

Theorem 10.1. *(Benjamini and Hochberg (1995)[22]) Consider tests of n null hypotheses, n_0 of which are true. If the test statistics (or equivalently, p-values) of these tests are independent, then the FDR of the above procedure satisfies*[23]

$$\mathrm{FDR} \leq \frac{n_0 q}{n} \leq q.$$

Motivation of the BH procedure: For each $\alpha \in (0,1)$, let $R(\alpha)$ be the number of p-values $\leq \alpha$. If we reject hypotheses with p-value $\leq \alpha$, then we expect (on average) to falsely reject αn_0 null hypotheses, since the null p-values are distributed as Uniform $(0,1)$. So we might estimate the false discovery proportion by

$$\alpha n_0 / R(\alpha)$$

As we don't know n_0, let's take the conservative upper-bound

$$\alpha n / R(\alpha)$$

If we set $\alpha = P_{(r)}$, the r th largest p-value, then $\alpha n / R(\alpha) \leq q$ exactly when $P_{(r)} \leq qr/n$. So the BH procedure chooses α (in a data-dependent way) so as to reject as many hypotheses as possible, subject to the constraint $\alpha n / R(\alpha) \leq q$.

Proof. Let's prove (more formally) the theorem that BH controls the FDR. For any event $\mathcal{E}$, we use the indicator notation

$$\mathbb{1}\{\mathcal{E}\} = \begin{cases} 1 & \mathcal{E} \text{ holds} \\ 0 & \mathcal{E} \text{ does not hold.} \end{cases}$$

[22] Benjamini, Y., & Hochberg, Y. (1995). Controlling the false discovery rate: a practical and powerful approach to multiple testing. Journal of the Royal Statistical Society: series B (Methodological), 57(1), 289-300.

[23] FDR control is not guaranteed if the test statistics are dependent.

Without loss of generality, order the n null hypotheses $H_0^{(1)}, \ldots, H_0^{(n)}$ so that the first n_0 of them are true nulls. Then

$$\begin{aligned}
\mathrm{FDR} &= \mathbb{E}[\mathrm{FDP}] \\
&= \mathbb{E}\left[\sum_{r=1}^{n} \frac{V}{r} \mathbb{1}\{R = r\}\right] \\
&= \mathbb{E}\left[\sum_{r=1}^{n} \sum_{j=1}^{n_0} \mathbb{1}\left\{\text{ reject } H_0^{(j)}\right\} \frac{1}{r} \mathbb{1}\{R = r\}\right],
\end{aligned}$$

(where we have noted $V = \sum_{j=1}^{n_0} \mathbb{1}\left\{\text{reject } H_0^{(j)}\right\}$).

Applying linearity of expectation,

$$\begin{aligned}
\mathrm{FDR} &= \sum_{r=1}^{n} \sum_{j=1}^{n_0} \frac{1}{r} \mathbb{E}\left[\mathbb{1}\left\{\text{ reject } H_0^{(j)}\right\} \mathbb{1}\{R = r\}\right] \\
&= \sum_{r=1}^{n} \sum_{j=1}^{n_0} \frac{1}{r} \mathbb{P}\left[\text{ reject } H_0^{(j)} \text{ and } R = r\right].
\end{aligned}$$

For fixed j, let $P_{(1)}^* \leq \ldots \leq P_{(n-1)}^*$ be the sorted $n-1$ p-values other than P_j. Then the BH procedure rejects r total hypotheses including $H_0^{(j)}$ if and only if $P_j \leq \frac{qr}{n}$ and the following event holds:

$$\mathcal{E}^{(r)} := \left\{P_{(1)}^*, \ldots, P_{(r-1)}^* \leq \frac{qr}{n},\ P_{(r)}^* > \frac{q(r+1)}{n},\ P_{(r+1)}^* > \frac{q(r+2)}{n}, \ldots,\ P_{(n-1)}^* > q\right\}.$$

As the p-values are independent, P_j is independent of $P_{(1)}^*, \ldots, P_{(n-1)}^*$. Furthermore, $P_j \sim \mathrm{Uniform}(0,1)$. So

$$\begin{aligned}
\mathrm{FDR} &= \sum_{r=1}^{n} \sum_{j=1}^{n_0} \frac{1}{r} \mathbb{P}\left[P_j \leq \frac{qr}{n} \text{ and } \mathcal{E}^{(r)} \text{ holds}\right] \\
&= \sum_{j=1}^{n_0} \sum_{r=1}^{n} \frac{1}{r} \mathbb{P}\left[P_j \leq \frac{qr}{n}\right] \mathbb{P}\left[\mathcal{E}^{(r)} \text{ holds}\right] \\
&= \sum_{j=1}^{n_0} \sum_{r=1}^{n} \frac{1}{r} \frac{qr}{n} \mathbb{P}\left[\mathcal{E}^{(r)} \text{ holds}\right] = \frac{q}{n} \sum_{j=1}^{n_0} \sum_{r=1}^{n} \mathbb{P}\left[\mathcal{E}^{(r)} \text{ holds}\right].
\end{aligned}$$

Finally, note that (for any fixed j) the events $\mathcal{E}^{(1)}, \ldots, \mathcal{E}^{(n)}$ are mutually exclusive – $\mathcal{E}^{(r)}$ holds if and only if the largest index k such that $P_{(k)}^* \leq \frac{q(k+1)}{n}$ is exactly $k = r-1$ (with $\mathcal{E}^{(1)}$ holding if $P_{(k)}^* > \frac{q(k+1)}{n}$ for all k), and this is true for exactly one value of $r \in \{1, \ldots, n\}$. So

$$\sum_{r=1}^{n} \mathbb{P}\left[\mathcal{E}^{(r)} \text{ holds}\right] = 1.$$

Hence

$$\mathrm{FDR} \leq \frac{q}{n} \sum_{j=1}^{n_0} 1 = \frac{qn_0}{n} \leq q.$$

□