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What is randomness?

In applied sciences one can consider lack of predictability:

Deterministic result For example, a medical treatment cures all patients, but without treatment no one gets cured.

Random result For example, when treated, 60 % gets cured but without treatment 20 % gets cured.

Data analysis with R software Random variable

Other examples of randomness

- Coin tossing or dice throwing
- Quantum mechanics
- Weather
- Stock market

Data analysis with R software

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Some examples on definitions of probability

A **unique event** cannot be predicted unless it is certain. One can form **subjective probabilities** prior to the observation.

If the process, which generates the data, can be **repeated**, the frequencies of different events can be calculated \Rightarrow **frequency probabilities**.

Continuous vs. discrete random variables

- A discrete random variable can have a countable number of values¹.
 A single value can have a positive probability. For example,
 - Dice throwing (6 possible values)
 - Number of heads before the first tail in coin tossing (infinite number of possible values 0, 1, 2, ...)
- A continuous random variable has a zero probability for any single value. For example,
 - Height of a person.
 - Blood pressure.

¹The values can be enumerated 1, 2, 3, ...

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Characterization of probabilities

Cumulative distribution function The probability that a random variable X gets a value less or equal to x: $\mathbb{P}\{X \le x\}$. For example, the probability that the height of a randomly chosen subject in the classroom is at most x = 170 cm.

Density function The point probability that a **discrete** random variable X equals some constant value x can be zero or positive. For example, the probability that a randomly chosen subject in the classroom is x = male. For a continuous random variable, value of a density function at x multiplied by a small positive constant a > 0is approximately the probability that the value of the r.v. is within [x, x + a]. Data analysis with R software Functions for random variables in R

Distributions

- See help page Distributions for complete list.
- Generally four functions available for each distribution starting with letters
 - d Density function
 - p Qumulative distribution function
 - q Quantile function
 - r Random number generation
- For example, normal distribution has functions

```
dnorm(x, mean = 0, sd = 1, log = FALSE)
pnorm(q, mean = 0, sd = 1, lower.tail = TRUE, log.p = FALSE)
qnorm(p, mean = 0, sd = 1, lower.tail = TRUE, log.p = FALSE)
rnorm(n, mean = 0, sd = 1)
```

Normal distribution

pdf("normal_dist.pdf", width = 7, height = 3.5)
x <- seq(-3, 3, 0.01)
plot(x, dnorm(x, mean = 0, sd = 1), type = "1", col = "red",
 lwd = 2, ylim = c(0, 1), ylab = "")
lines(x, pnorm(x, mean = 0, sd = 1), type = "1", col = "blue",
 lwd = 2)
legend("topleft", lty = c(1, 1), lwd = 2, col = c("red", "blue"),
 cex = 1, legend = c("Density function", "Cumulative distribution function"))
dev.off()</pre>



Data analysis with R software

Discrete uniform distribution

- > Observations are between a minimum and maximum integer values
- ► All intervals of the same length on the distribution's support {min, min+1,..., max} are equally probable.

Example In dice rolling minimum is 1 and maximum 6:



Continuous uniform distribution

- Observations are between a minimum and maximum (a.s.)
- All intervals of the same length on the distribution's support [min, max] are equally probable.



Data analysis with R software

Sum of two discrete uniformly distributed random variables

Example Assume rolling two independent dices X_1 and X_2 (min=1 and max=6).

What is the probability of the sum $S := X_1 + X_2$ being equal to s?



• 6×6 different outcomes $(X_1, X_2) = (x_1, x_2)$ with equal probabilities 1/36

- The sum s is between a 2 \times minimum and 2 \times maximum
- Maximum probability is at s = 7: $\mathbb{P}{S = 7} = 6/36 \approx 0.167$

Central limit theorem (CLT)

Consider a sum of independent random variables.

The more terms there are in the sum, the closer the distribution of the sum resembles normal distribution.

Example: random variables with uniform distribution on [-1, 1].

Sum of 2 uniformly distributed r.v. 's



Data analysis with R software Repeated experiments

An experiment

- A researcher has a hypothesis.
- He/she plans and executes an experiment, and collects data in order to test the validity of the hypothesis.
- Question: Does the data support the hypothesis?
- For example, coin tossing:
 - Hypothesis is "Probability of heads in is 0.5."
 - Experiment is "Toss a coin 10 times."
 - Data are the proportion of heads.
- ► For example, medical experiment:
 - Treatment group and control group.
 - ► Null hypothesis, H0: "No difference between groups" i.e. the treatment has no effect.
 - > Alternate hypothesis, H1: "Some differences between groups."
 - Experiment is "Administer the treatment to the treatment group and some placebo treatment to the control group."
 - Data are the recovery status of the patients (and the group indicator).

Data analysis with R software Central limit theorem

What does CLT mean in practice?

Many estimators are sums over observed values, which are independent. E.g. sample mean

$$\bar{x} = \frac{1}{n} \sum_{i=1}^{n} x_i$$

Therefore sampling distributions of estimators are often normal, if the sample size is large.

Data analysis with R software Repeated experiments

Experiments should be repeatable

- Other researchers should be able to repeat the experiment in (identical) conditions.
- In frequentist inference one assumes (infinitely) many hypothetical experiments, in which the null hypothesis is true.
- The outcome of the observed experiment is then compared with the distribution of the outcomes of the hypothetical experiments.
- If the observed outcome seems to be very rare, then the empirical evidence does not support the null hypothesis.
- ► Observational vs. experimental studies: observational studies can be unique ⇒ the idea of repeated experiments (or new samples of study subjects) in identical conditions unrealistic.

Distribution of hypothetical experiments

Return to the experiment with 10 tosses of a coin. 3 heads were observed.

- **H0**: Probability of head is 0.5.
- The probability distribution of number of heads in 10 tosses is Binomial.

```
x <- 0:10
```

pdf("binom10.pdf", width = 7, height = 3.5)
plot(x, dbinom(x, size = 10, p = 0.5), type = "h", xlab = "Number of heads",





Hypothesis testing Binary variable

Example: binomial test

The experiment with 10 tosses of a coin. 3 heads were observed.

Note that the expected number of heads is $10\times0.5=5$ and the sampling distribution is symmetric.

The p-value of the one-sided test is 0.17 and of the two-sided test 0.34.



One- and two-sided tests

"How often hypothetical experiments would generate data at least as rare as the observed outcome?"

One-sided test Calculate the probability of sampling distribution at the observed value and at the more extreme values.

Two-sided test If the sampling distribution is symmetric, calculate the p-value from both tails of sampling distribution.

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Sample size of the experiment

The researcher considers the coin to be "unfair" if the probability of head is outside [0.45, 0.55].

- Are 10 tosses enought to detect the bias?
- If not, then how many are needed?

Recall that the standard error (SE) of the estimated proportion is

$$\mathsf{SE}(\hat{p}) = \sqrt{\frac{p \times (1-p)}{n}}.$$
 (1)

With n = 10 tosses and p = 0.5, SE(\hat{p}) = 0.16.

Approximately 95 % of the sampling distribution lies between $p \pm 1.96 \times SE(\hat{p})$. Desired sample size can be solved from (1): $n = (1.96^2/a^2) \times p \times (1-p)$, where a = 0.05 is the desired accuracy.

The researcher decides to toss the coin n = 384 times.

Mean of normally distributed r.v.'s

SD is known

Assume that *n* independent r.v.'s are sampled from N(μ , σ^2). Recall that parameter μ is expectation and σ^2 is variance (σ is standard deviation SD).

The sample mean \bar{x} is also normally distributed N(μ , σ^2/n). The SE of the sample mean is σ/\sqrt{n} . Large $n \Rightarrow$ small SE.

Example: n = 16, $\sigma^2 = 4$ and $\bar{x} = 3$. Let the null hypothesis be $H_0: \mu = \mu_0 = 2$. Then test statistic is

$$z = \frac{\bar{x} - \mu_0}{\sigma / \sqrt{n}} = \frac{3 - 2}{2/4} = 2.$$

The tail probability of one-sided test is

1 - pnorm(2, mean=0, sd=1) = 0.023.

```
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```

Student's t test

SD is unknown

Typically σ is unknown as well as μ . If the sample size *n* is large then the sample SD s is close to the true σ .

If n is small, then the uncertainty of s needs to be accounted for using t distribution with n-1 degrees of freedom (df) instead of normal distribution.

Example: n = 16, $s^2 = 4$ and $\bar{x} = 3$. Let the null hypothesis be H_0 : $\mu = \mu_0 = 2$. Then test statistic is

$$t = \frac{\bar{x} - \mu_0}{s/\sqrt{n}} = \frac{3-2}{2/4} = 2.$$

The tail probability of one-sided test is 1 - pt(2, df=16-1) = 0.032.

Data analysis with R software Hypothesis testing Continuous variable

The t distribution vs. normal distribution

If n is small, then s is imprecise, thus the sampling distribution contains more extreme values than normal distribution. The smaller degrees of freedom (df), the more extreme values. If df (and n) are large, then t distribution is close to normal distribution (s is close to σ).



Data analysis with R software
Hypothesis testing
└─ Independent two-sample tests

Comparing two independent samples: t test

SD unknown, possibly unequal

Test statistic is now

mean of x mean of y 5.936

6.588

$$t = \frac{\bar{x_1} - \bar{x_2}}{\sqrt{\frac{s_1^2}{n_1} + \frac{s_2^2}{n_2}}}.$$

Notations are analogous to the one sample case. Example:

with(iris, t.test(Sepal.Length[Species == "versicolor"], Sepal.Length[Species == "virginica"]))

Welch Two Sample t-test ## ## data: Sepal.Length[Species == "versicolor"] and Sepal.Length[Species == "virginica" ## t = -5.629, df = 94.03, p-value = 1.866e-07 ## alternative hypothesis: true difference in means is not equal to 0 **##** 95 percent confidence interval: ## -0.882 -0.422 ## sample estimates:

Samples are not normally distributed

Some non-parametric tests

Median test Calculate the **median** of the joined data sets, create a binary variable and do the χ^2 test for the 2 × 2 table.

Mann-Whitney test More efficient than the median test. Does not assume normality. Based on ranks of observations. Install package exactRankTests, where the function wilcox.exact can be found.

Data analysis with R software
Hypothesis testing
Paired two-sample tests

Two measurements on the same subjects

Any changes between measurements?

For each subject *i* there are two measurements x_i and y_i . For example, a measurement before medical treatment and the other after the treatment.

- > The measurements are often correlated.
- Positive correlation means e.g. that subjects *i* who had high level of symptoms at the time of the first measurement x_i, tend to have high level of symptoms also at the second measurement y_i.
- ▶ The correlation must be accounted for in analyses.

Data analysis with R software Hypothesis testing Paired two-sample tests

Paired two-sample tests

The arguments are generally two vectors of the same length.

The t test Use the **t.test** function and **paired=TRUE** argument.

- Sign test Calculate the number of pairs *i* for which $x_i < y_i$. This number should be close to 50 % of the number of pairs. Compare with Binomial distribution as with the binomial test above.
- Wilcoxon test More efficient than the sign test. Does not assume normality, but requires a symmetric distribution. (If distribution is not symmetric, then use the sign test.) Install package exactRankTests, where the function wilcox.exact can be found. Use option paired=TRUE.