Medical Statistics

2022-06-14

Contents

٨	hout	this course 5
А		chers
		ls & Topics
		requisites
		erials
		gramme
	1108	ramme
1	Inti	oduction and exploratory data analysis 9
	1.1	Introduction to probabilistic distributions
	1.2	Data representation
	1.3	Mean-Variance
	1.4	Correlation and heatmap
2	Sta	tistical tests - Part 1
_	2.1	Comparing 2 groups
	2.2	Power of a statistical test
3	Cor	nmon regression models 13
•	3.1	Linear regression
	3.2	Logistic regression
4	Sto	tistical tests - Part 2
4	4.1	Comparing more than two groups
	4.1	
	4.2 4.3	8
	$\frac{4.5}{4.4}$	8
	$\frac{4.4}{4.5}$	Testing symmetry in 2x2 tables
	$\frac{4.5}{4.6}$	
	4.0	Power for analysis of count tables
5	\mathbf{Sur}	vival analysis 19
	5.1	Survival data - Introduction
	5.2	Survival data - regression models
	5.3	Survival data - power analysis
	5.4	Survival analysis - Notes

4	CONTENTS

6	$\mathbf{E}\mathbf{x}\mathbf{e}$	ercises																	2
	6.1	Chapter 1																	2
	6.2	Chapter 2																	2
	6.3	Chapter 3																	2
	6.4	Chapter 4																	2
	6.5	Chapter 5																	2
7	App	oendix																	2
	71	PCA Anal	vc	ic															9

About this course

In this course an introduction to basic statistical methods useful for biomedical data analysis will be given. During the course we will alternate between lectures and practicals, allowing for plenty of interaction and illustration with examples of practical interest.

The course requires little prior statistics knowledge, but assumes participants are able to work with R, R packages and RMarkdown for the practicals. Participants with no or little experience in R are strongly advised to follow an introductory R course prior to following this course, such as the one we offer.

The course program includes: exploratory data analysis, basic statistical tests, methods for count data tables, linear and logistic regression, as well as basic methods for survival data analysis. For each method, power analysis and sample size determination will be handled.

Teachers

- Renee Menezes, Biostatistics Center, NKI (coordinator)
- Renaud Tissier, Biostatistics Center, NKI
- Leyla Azarang, Biostatistics Center, NKI

Goals & Topics

After the course you will be able to:

- Perform data exploration analysis
- Perform comparison between two different groups using statistical tests
- Perform linear and logistic regression
- Perform survival data analysis
- Use the R software to perform these tasks

We will cover the following topics:

- Probabilistic distributions
- Hypothesis testing

6 CONTENTS

- Linear regression
- Logistic regression
- Survival data analysis
- Power analysis

Prerequisites

The course assumes no prior programming knowledge. Elementary statistics knowledge is necessary.

Participants must bring own laptops capable of running RStudio.

Before the course **please prepare your laptop**:

- 1. install R, an open-source, free environment for statistical computing and graphics. You can find instructions for downloading and installing it from one of the CRAN mirrors, for example from the Univ. of Gent or from the Imperial College. A full list of mirrors can be found here.
- 2. install RStudio. Go to the RStudio download page, select a version of RStudio appropriate for your laptop, download it and then install. Please check whether you can start RStudio.
- 3. install RMarkdown, a very nice and easy tool to produce reports using RStudio. It is made available as an R package for Rstudio. One easy way to install it is as follows:
- i) open RStudio
- ii) click on the "File" menu on the top left, and choose "New file">"R Markdown". If RMarkdown is not yet installed on your machine, this will prompt you to install it and any packages required. Just follow the instructions that appear on the screen.
- 4. Several packages are required to perform the course. These packages can be installed before the course to gain some time. The required packages are:
- factoextra
- gplots
- pwr
- statmod

Materials

After the course material .zip file is downloaded, the course material can be assessed:

• as HTML pages by opening index.html in any browser

CONTENTS 7

- by clicking on the ${\tt RcourseNKI.Rproj}$ file, which will open the entire course as an R project

• via the course source git repository

The materials contain a data directory with the data files used in the presentations/tasks. The directory can be also accessed at https://github.com/rxmenez es/RcourseNKI/tree/master/data

Programme

Third NKI edition, November 22nd, 23rd, 24th, 25th and 26th 2021

This course will be given online via Zoom. All course days are in the period 9:00-16:00, with the last hour reserved for general Q&A.

8 CONTENTS

Introduction and exploratory data analysis

- 1.1 Introduction to probabilistic distributions
- 1.1.1 Motivation
- 1.1.2 Discrete random variables
- 1.1.3 Continuous random variable
- 1.2 Data representation
- 1.2.1 Motivating example
- 1.2.2 Working example
- 1.3 Mean-Variance
- 1.3.1 Working example
- 1.4 Correlation and heatmap
- 1.4.1 Motivation
- 1.4.2 Working example

10CHAPTER 1. INTRODUCTION AND EXPLORATORY DATA ANALYSIS

Statistical tests - Part 1

- 2.1 Comparing 2 groups
- 2.1.1 Motivation
- 2.1.2 Working Example
- 2.2 Power of a statistical test
- 2.2.1 Motivation
- 2.2.2 Working example

Common regression models

- 3.1 Linear regression
- 3.1.1 Motivation
- 3.1.2 Working Example
- 3.2 Logistic regression
- 3.2.1 Motivation
- 3.2.2 Link function
- 3.2.3 The glm function
- 3.2.4 Working example
- 3.2.5 Fitted values

Statistical tests - Part 2

4.1 Comparing more than two groups

- 4.1.1 Motivation
- 4.1.2 Working examples
- 4.2 Testing independence in 2x2 tables
- 4.2.1 Motivation
- 4.2.2 Working examples
- 4.2.3 General setup
- 4.2.4 Working examples (cont)
- 4.2.5 The chi-square test
- 4.2.6 Working examples (cont)
- 4.2.7 Fisher's exact test
- 4.2.8 Working examples (cont)
- 4.3 Testing independence in nx2 tables
- 4.3.1 Motivation
- 4.3.2 Working examples
- 4.3.3 General setup
- 4.3.4 Chi-square test for nx2 tables
- 4.3.5 Working examples (cont)
- 4.4 Testing symmetry in 2x2 tables
- 4.4.1 Motivation
- 4.4.2 Working example
- 4.4.3 General setup
- 4.4.4 Working example (cont)
- 4.4.5 Notes
- 4.5 Relative risk and odds ratio
- 4.5.1 Motivation
- 4.5.2 Relative risk
- 4.5.3 Odds ratio
- 4.5.4 Relative risk vs. odds ratio
- 4.5.5 Logistic regression models (cont)
- 4.5.6 Poisson regression models

Survival analysis

5.1 Survival data - Introduction

- 5.1.1 Motivation
- 5.1.2 Working example
- 5.1.3 Kaplan-Meier curve
- 5.1.4 Working example (cont)
- 5.1.5 Survival data analysis in R
- 5.1.6 Survival data for groups: the log-rank test
- 5.1.7 Working example (cont)
- 5.2 Survival data regression models
- 5.2.1 Motivation
- 5.2.2 Cox proportional-hazards model
- 5.2.3 Working example
- 5.2.4 Group-specific baseline hazards
- 5.2.5 The proportional hazards assumption
- 5.2.6 Others
- 5.3 Survival data power analysis
- 5.3.1 Motivation
- 5.3.2 Power and sample size for Cox regression
- 5.3.3 Working example
- 5.3.4 Minimum sample size
- 5.3.5 Working example (cont)
- 5.3.6 Reference
- 5.3.7 Notes
- 5.4 Survival analysis Notes
- 5.4.1 Other types of censoring
- 5.4.2 Right-, left- and interval censoring
- 5.4.3 Type I and type II censoring
- 5.4.4 Competing risks

Exercises

- 6.1 Chapter 1
- 6.1.1 Exercise 1
- 6.1.2 Exercise 2:
- 6.1.3 Exercise 3(*)
- 6.2 Chapter 2
- 6.2.1 Exercise 1
- 6.2.2 Exercise2
- 6.3 Chapter 3
- 6.3.1 Exercise 1
- 6.3.2 Exercise 2
- 6.3.3 Exercise 3
- 6.4 Chapter 4
- **6.4.1** Exercise 1
- 6.4.2 Exercise 2
- 6.4.3 Exercise 3
- 6.4.4 Exercise 4
- 6.5 Chapter 5
- 6.5.1 Exercise 1
- 6.5.2 Exercise 2
- 6.5.3 Exercise 3

Appendix

- 7.1 PCA Analysis
- 7.1.1 Motivation
- 7.1.2 Working example
- 7.1.3 Quick tasks