

Study Guide

## Principles of Statistical Inference

Semester 1, 2023

Prepared by: Liz Barnes NHMRC Clinical Trials Centre University of Sydney

Dr Erin Cvejic Sydney School of Public Health University of Sydney

Copyright © University of Sydney



## Contents

2
2
2
2
3
3
3
3
1
5
5
7
3
3
9
9
)
)
)
1
1

## Contact details

Liz Barnes NHMRC Clinical Trials Centre Faculty of Medicine and Health University of Sydney Email: Liz.Barnes@sydney.edu.au Phone: (02) 9562 5303 (however please contact by email only)

Liz is a Biostatistician and Research Fellow at the NHMRC Clinical Trials Centre, University of Sydney. She is responsible for both the content and administration of the unit. One or more other biostatisticians from the Clinical Trials Centre assist throughout the semester with involvement in online discussions and marking of assessments.

If you have any general BCA queries, please contact: Karolina Kulczynska-Le Breton or Emily Higginson at the BCA Coordinating Office on 02 9562 5076/54 or via email to <u>bca@sydney.edu.au</u>

## Background

A sound understanding of the basic principles of statistical inference, including the theory of statistical estimation and hypothesis testing, is necessary for students to gain a deeper understanding of methods used in the design and analysis of biomedical and epidemiological studies. Specifically, it verses students in the language of uncertainty. An understanding of the theoretical bases and drawbacks of common biostatistical techniques is essential for practising biostatisticians to be able to assess the validity of these techniques for particular studies, and to be able to modify those techniques where appropriate. In this unit of study (unit) students will develop a strong mathematical and conceptual foundation in the methods of statistical inference, which underlie many of the methods utilised in subsequent units of study, and in biostatistical practice.

### Context within the program

This unit will develop a strong mathematical and conceptual foundation in the methods of statistical inference, with a particular focus on likelihood-based methods. These concepts and principles will underlie many of the methods utilised in subsequent units of study, as well as biostatistical practice in general.

#### Prerequisites:

Mathematics Foundations for Biostatistics (MFB);

OR

Mathematical Background for Biostatistics (MBB) AND Probability and Distribution Theory (PDT)

## **Unit summary**

The unit provides an overview of the concepts and properties of estimators of statistical model parameters, then proceeds to a general study of the likelihood function from first principles. This will serve as the basis for likelihood-based methodology, including maximum likelihood estimation, and the likelihood ratio, Wald, and score tests. Core statistical inference concepts including estimators and their ideal properties, hypothesis testing, p-values, confidence intervals, and power under a frequentist framework will be examined with an emphasis on both their mathematical derivation, and their interpretation and communication in a health and medical research setting. Other methods for estimation and hypothesis testing, including a brief introduction to the Bayesian approach to inference, exact and non-parametric methods, and simulation-based approaches will also be explored.

## Workload requirements

The expected workload for this unit is **10-12 hours per week on average**, consisting of textbook readings, discussion board posts, independent study, tutorial sessions and completion of assessment tasks.

## **Learning Outcomes**

At the completion of this unit students should be able to:

- 1. Calculate and interpret important properties of point and interval estimators
- 2. Calculate and interpret p-values, power and confidence intervals correctly
- 3. Write a likelihood function
- 4. Derive and calculate the maximum likelihood estimate
- 5. Derive and calculate the expected information
- 6. Derive a Wald test, Score test, and likelihood ratio test
- 7. Use a Bayesian approach to derive a poster distribution
- 8. Calculate and interpret posterior probabilities and credible intervals
- 9. Apply and explain an exact method, non-parametric and sampling-based method

## Unit content

The unit is divided into 6 modules, summarised in more detail below. Each module will involve approximately 2 weeks of study and generally includes the following material:

1. Recorded lectures on the theory content

2. One or two chapters from the textbook, which includes statistical theory and extended examples illustrating the statistical theory covered

- 3. A recorded lecture covering the extended example
- 4. Practical exercises, one of which is required to be submitted for assessment
- 5. Worked written and video solutions to non-assessed practical exercises

6. Discussion boards which can be used to ask questions and discuss module content and non-assessed exercises

7. Tutorial sessions for each module

With the exception of the textbook, study materials for all modules are downloadable from the eLearning (Canvas) unit site. Assignments and supplementary material, such as analysis datasets, will be posted to the unit site. Please note that we are not able to post copies of copyright material (journal articles and book extracts)—for these you will have to rely on your home university's library.

## **Recommended approaches to study**

Students should begin each module by watching the lecture recording, reading through the relevant chapter of the textbook, and working through the extended example in parallel with the exercises. You are encouraged to post any content-related questions to eLearning, whether they relate directly to a given exercise, or are a request for clarification or further explanation of an area in the notes. You should also work through any computational examples in the notes for yourself on your own computer.

Solutions to the exercises in each module (except those to be submitted for assessment, as described below) will be posted online after the midway point of the allocated time period for the module. This is intended to encourage you to attempt the exercises independently before being given access to solutions.

Some of the exercises require computer simulations, and for these Stata and R code will be provided on eLearning. You are welcome to use any other software you have available and are familiar with for the exercises (e.g., SAS, Matlab, Python), however code will not be provided for these packages and assistance may not be available.

Some exercises require the creation of graphs – these can be done in statistical software or a spreadsheet package (e.g. Excel) and must comply with the guidelines for reporting of statistical results found on the <u>BCA student resources Canvas site</u>.

Although a nominal period of 14 days is allocated to work on each module, students can ask questions about the material in any previous modules at any time during the semester.

Make the most of this unit by engaging with coordinators and fellow students on the Discussion Board and in Tutorials. These are safe spaces to discuss the course material and related ideas and students are encouraged to make the most of them by engaging in respectful discussion.

Questions about Assignments should be directed to the coordinator in the first instance to avoid any Academic Honestly issues.

## Method of communication with coordinator(s)

The eLearning website is the primary forum for communication between coordinators and students. It will also be used for posting all course material. The timetable below shows the dates when assignments will be made available and when all assessments are due. Please check the Canvas site regularly for new material and to keep up-to-date with class discussions.

Please post content-related questions to the relevant Discussion forum in the PSI eLearning site. You should be familiar with the eLearning system from previous BCA units, and will receive any specific instructions on using the eLearning site this semester from the BCA Coordinating Office. There is also useful available on the <u>BCA student</u> resources Canvas site.

Questions about administrative aspects or course content can be emailed to the coordinator. Please use "PSI" in the Subject line of your email to assist in keeping track of our email messages. Coordinator/s will be available to answer questions related to the module notes and practical exercises, and to address any other issues that require clarification.

Please note that instructors are not necessarily available every day of the week and you should expect that it may take a day or so to respond to questions (possibly longer over weekends, during breaks, and NSW public holidays).

We strongly recommend that you post content-related questions to the Discussion Board in the unit site. For matters of a personal nature, please contact the unit of study coordinator directly by email. Email is recommended to make initial contact and then a teleconference via Zoom can be arranged at a convenient time if required.

## **Module descriptions**

Below is an outline of the study modules, followed by a timetable and assessment description table. Each module of this unit corresponds to a chapter in unit textbook. Each module is scheduled to begin on a Monday and conclude on the Sunday of the following week. The due date for submission of the required exercises from each module is 11:59PM (Sydney Time) on the due date indicated below.

#### Module 1: Estimation concepts (Chapter 2)

- Probability models and parameters
- Estimates and estimators
- Point and interval estimation
- Bias and efficiency
- Consistency and asymptotic efficiency

#### Module 2: Hypothesis testing concepts (Chapter 5)

- Null and alternative hypotheses
- Test statistics
- P-values
- Type I and Type II errors, significance level, and power
- Statistical significance and practical importance

#### Module 3: Likelihood and Estimation Methods (Chapters 3 and 4)

- Likelihood function
- Sufficiency
- Nuisance parameters
- Maximum likelihood estimation
- Statistical information
- Properties of maximum likelihood estimation

#### Module 4: Hypothesis testing methods (Chapter 6)

- Likelihood ratio tests
- Score tests
- Wald tests
- Relationship between the three tests
- Interval estimation based on the three tests

#### Module 5: Bayesian methods (Chapter 7)

- Basic concepts: subjective probability
- Bayes' rule, prior and posterior distributions
- Conjugate and non-informative prior distributions
- Analysis of simple binomial and normal models

#### Module 6: Further inference methods (Chapter 8)

- Exact methods
- Non-parametric methods
- Bootstrapping and other resampling methods

## Unit schedule

Week	Week commencing Monday	Module	Торіс	Assessment and due date		
1	27 <sup>th</sup> February	Module 1	Estimation concepts	M1 exercise due		
2	6 <sup>th</sup> March			Sunday 12 <sup>th</sup> March		
3	13 <sup>th</sup> March	Module 2	Hypothesis testing concepts	M2 exercise due Sunday 26 <sup>th</sup> March		
4	20 <sup>th</sup> March			Sunday 20 March		
5	27 <sup>th</sup> March	Module 3	Likelihood and estimation methods	Assignment 1 released Friday 7 <sup>th</sup> April 5pm		
6	3 <sup>rd</sup> April			M3 exercise due Sunday 9 <sup>th</sup> April		
7	10 <sup>th</sup> April	Mid-semester break (Revision and assignment)				
8	17 <sup>th</sup> April	Module 4	Hypothesis testing methods	Assignment 1 Due Sunday 23 <sup>rd</sup> April		
9	24 <sup>th</sup> April			M4 exercise Due Sunday 30 <sup>th</sup> April		
10	1 <sup>st</sup> May	Module 5	Bayesian inference	M5 exercise due		
11	8 <sup>th</sup> May			Sunday 14 <sup>th</sup> May		
12	15 <sup>th</sup> May	Module 6	Further inference methods	Assignment 2 Released Friday 26 <sup>th</sup> May 5pm		
13	22 <sup>nd</sup> May			M6 exercise due Sunday 28 <sup>th</sup> May		
14	29 <sup>th</sup> May	Revision and assignment		Assignment 2 Due		
	5 <sup>th</sup> June			Sunday 11 <sup>th</sup> June		

Semester 1, 2023 starts on Monday 27<sup>th</sup> February

### Assessment

Assessment will include 2 written assignments worth 40% each, to be made available in the middle and at the end of the semester, and to be completed within approximately two weeks (including three weekends). These assignments will be posted on the eLearning site together with an online Announcement broadcasting their availability. In addition, students will be required to submit solutions to selected practical exercises (one from each module), worth a total of 20%, by deadlines specified throughout the semester (see table below).

Assessment name	Assessment type	Coverage	Learning objectives	Weight
Module 1 exercises	Assignment	Module 1	1,2	4%*
Module 2 exercises	Assignment	Module 2	1,2	4%*
Module 3 exercises	Assignment	Module 3	1,2,3,4,5	4%*
Assignment 1	Assignment	Modules 1-3	1,2,3,4,5	40%
Module 4 exercises	Assignment	Module 4	1,2,3,4,5,6	4%*
Module 5 exercises	Assignment	Module 5	1,2,3,4,7,8	4%*
Module 6 exercises	Assignment	Module 6	1,2,3,4,5,9	4%*
Assignment 2	Assignment	Modules 1-6	1,2,3,4,5,6,7,8,9	40%

# \* Your best five modules from six will each contribute 4% each towards the total 20% for the module exercises.

You are required to submit work typed in Word (or similar). We strongly recommend you become familiar with equation typesetting software such as Microsoft's Equation Editor for algebraic work. See the <u>BCA Assessment Guide</u> for guidelines on acceptable standards for assessable work.

Students are encouraged to discuss relevant topics in the Discussion Board. However, please avoid posting questions relating directly to assessable material. These should be emailed to the Unit Coordinator in the first instance.

*Explicit solutions to assessable exercises should not be posted for others to use.* Each student's submitted work must be clearly their own, with anything derived from other students' discussion contributions clearly attributed to the source.

#### Submission and academic honesty policy

All assessment material should be submitted via the relevant Assessment module in Canvas unless otherwise advised. Turnitin plagiarism detection is applied to all submissions. For detailed information, please see the <u>BCA Assessment Guide</u>, which includes links to the Academic Honesty policies at member universities. Please familiarise yourself with the procedures and policies at your home university. You will need to indicate your compliance with the plagiarism guidelines and policy at your home university.

A special note regarding "contract cheating" sites: Unfortunately, there have been instances in the past of students using such websites to post assignment questions and receive solutions (usually for a fee). We have arrangements with these sites to identify the student posting questions or accessing the solutions, and such students will be referred to and face disciplinary processes at their home university.

#### Late submission and extension procedure

The standard BCA policy for late penalties for submitted work is a 5% deduction from the earned mark for each day the assessment is late, up to a maximum of 10 days (including weekends and public holidays). Extensions are possible, but these need to be applied for (by email) as early as possible. The Unit Coordinator can approve extensions up to three days; for extensions beyond three days, you must apply to your home university, using their standard procedures.

#### Learning resources

The textbook for this unit is:

Marschner, I.C. Inference Principles for Biostatisticians Chapman and Hall / CRC, 2014 ISBN 9781482222234 hard cover ISBN 9780367576011 paperback ISBN 9780429076244 eBook http://www.crcpress.com/product/isbn/9781482222234

**Note:** there are a small number of minor typographical errors in the chapters used in this unit; a list of these will be provided on the eLearning site – please take note of these before reading the relevant chapters.

This book contains all the material that will be covered in this unit of study. Note, that you *may* have digital access to this text through you home university library – check this before you purchase a copy.

Other references books which you may find useful include:

- 1. Ross S. A First Course in Probability. MacMillan, 1988. Background
- Azzalini A. Statistical Inference: Based on the Likelihood. Chapman and Hall, 1996. Modules 1 – 4.
- Clayton D and Hills M. Statistical Models in Epidemiology. Oxford University Press, 1993. Modules 1 – 4.
- Casella G and Berger RL. Statistical Inference. Wadsworth and Brooks/Cole, 1990. Modules 1-4.
- 5. McElreath R. Statistical rethinking: A Bayesian Course with Examples in R and Stan. Chapman and Hall / CRC, 2020. Module 5

- 6. Mood, A.M., Graybill, F.A. & Boes, D.C. (1963). Introduction to the theory of statistics (3rd ed.). McGraw-Hill. Modules 1 4.
- 7. Wackerley, D., Mendenhall, W., & Schaeffer RL. (2008). Mathematical Statistics with Applications. Wadsworth Group. Modules 1 4.

Additional readings (e.g., journal articles) may be recommended throughout the unit. Bibliographic details will be provided on Canvas.

## Software requirements and assumed knowledge

For this subject you will need to have access to either Stata or R. The purpose of this unit is not to teach statistical computing. However, there are some exercises that rely on the use of simulation to help understand the concepts being taught which can only be achieved by using software. Stata and R code will be provided on Canvas which can be modified as required for exercises and assessments.

If you have not used Stata or R previously, it is highly recommended that you attempt to familiarise yourself with it prior to the beginning of semester. If you have not yet organised access to these packages, you should do so as soon as possible. Information on how to download R and RStudio, and access Stata can be found in the <u>BCA</u><u>Textbook and Software Guides</u>.

Some students do struggle with the software elements of this unit. Please do not be afraid to ask for help from other students and instructors on Discussion Boards. Try not to allow any difficulties with software to obscure the basis of the course, which is to understand the principles of statistical inference. However, it is also important that practising biostatisticians can work in various software packages, so it is worthwhile making the effort to become proficient in at least one package.

## **Required mathematical background**

Students should be familiar with the mathematical background covered as part of MFB (or MBB), including basic factorisation, rules for handling exponents and natural logarithms, differentiation and partial differentiation, and basic matrix manipulations (i.e., inverse of a matrix).

### Feedback

#### Our feedback to you:

The types of feedback you can expect to receive in this unit are:

- Formal individual feedback on submitted exercises assignments
- Responses to questions posted on eLearning
- Informal feedback through discussion in Q+A sessions

#### Your feedback to us:

One of the formal ways students have to provide feedback on teaching and their learning experience is through the BCA student evaluations at the end of each unit. The feedback is anonymous and provides the BCA with evidence of aspects that students are satisfied with and areas for improvement. You are also more than welcome to contact the unit coordinator directly if you have feedback on the delivery of the unit, or the BCA office.

## Unit changes, including response to recent student evaluation

PSI is delivered in both Semester 1 and Semester 2 each year. From Semester 1 2022 there were major changes to some of the core units in the BCA program. PSI has added additional material in Module 1 - this material was previously covered in PDT and therefore may be revision for some students. Other PSI modules have also been rearranged.

Based on feedback from previous deliveries, we have introduced recorded video lectures to complement the textbook readings, recorded worked video solutions to the non-assessed module exercises to further reinforce concepts, and provide the opportunity for live consultation (either in the form of tutorial or Q&A sessions, depending on module content) via videoconferencing to increase engagement and interactivity with the teaching team.

## Acknowledgments

PSI has evolved over many deliveries, with valuable contributions from numerous colleagues and coordinators. The following people have been important in developing and delivering this unit and we thank them for their input:

- Dr Erin Cvejic
- Katrina Blazek
- Justin Zeltzer
- A/Prof Patrick Kelly
- Prof lan Marschner