

Study Guide

Principles of Statistical Inference

Semester 1, 2022

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Contact details

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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 may be assisting 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.

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, and completion of assessment tasks.

Prerequisites

Mathematic Background for Biostatistics (MBB) Probability and Distribution Theory (PDT)

PSI builds extensively upon the material covered in Probability and Distribution Theory (PDT). You may find it useful to refer back to your notes from PDT. The first chapter and the appendix of the textbook contain information that will be helpful for PSI – it is strongly recommended that you read that chapter early in the semester (or before) and refer to the appendix as required throughout the unit.

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. One or two chapters from the textbook, which includes statistical theory and an extended example illustrating the statistical theory covered
- 2. A recorded lecture(s) on the theory content
- 3. A recorded lecture going through the extended example.

- 4. Several practical exercises, one of which is required to be submitted for assessment.
- 5. A discussion board which should be used to ask questions about the module content and non-assessed exercises
- 6. A recorded video going through the solutions of non-assessed exercises, released mid-way through each module (along with the written solutions).

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.

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 at 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.

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 website 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, and when doing so please use "PSI" in the Subject line of your email to assist in keeping track of our email messages. Coordinators will be available to answer questions related to the module content and practical exercises, and to address any other issues that require clarification. However, 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).

For matters of a personal nature, please contact the unit of study coordinator directly. Due to working from home during the pandemic, email is recommended to make initial contact and then a phone call can be arranged at a convenient time.

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 1: 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

Semester 1,	2022 starts on	Monday 28 th	February
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Week	Week commencing Monday	Module	Торіс	Assessment and due date	
1	28 th February	Module 1	Estimation concepts		
2	7 th March	Module 1	Estimation concepts	M1 exercise due Sunday 13 th March	
3	14 th March	Module 2	Hypothesis testing concepts		
4	21 st March	Module 2	Hypothesis testing concepts	M2 exercise due Sunday 27 th March	
5	28 th March	Module 3	Likelihood and estimation methods		
6	4 th April	Module 3	Likelihood and estimation methods	Assignment 1 released Friday 8 th April 5pm M3 exercise due Sunday 10 th April	
7	11 th April	Module 4	Hypothesis testing methods		
8	18 th April	Mid semester break			
9	25 th April	Module 4	Hypothesis testing methods	Assignment 1 Due Tuesday 26th April M4 exercise Due Sunday 1 st May	
10	2 nd May	Module 5	Bayesian inference		
11	9 th May	Module 5	Bayesian inference	M5 exercise due Sunday 15 th May	
12	16 th May	Module 6	Further inference methods		
13	23 rd May	Module 6	Further inference methods	Assignment 2 Released Friday 27 th May 5pm M6 exercise due Sunday 29 th May	
14	30 th May				
	6 th June			Assignment 2 Due Sunday 12 th June	

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. 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 your work typed in Word or similar (e.g. using Microsoft's Equation Editor for algebraic work) and we strongly recommend that you become familiar with equation typesetting software such as this. If extensive algebraic work is involved you may submit neatly handwritten work, however please note that marks will potentially be lost if the solution cannot be understood by the markers due to unclear or illegible writing. This handwritten work should be scanned and collated into a single pdf file and submitted via the eLearning site. See the <u>BCA Assessment Guide</u> document for specific guidelines on acceptable standards for assessable work.

The instructors will generally avoid answering questions relating directly to the assessable material, but we encourage students to discuss the relevant parts of the notes via eLearning. **Explicit solutions to assessable exercises should not be posted for others to use**, and each student's submitted work must be clearly their own, with anything derived from other students' discussion contributions clearly attributed to the source.

Submission of assessments and academic honesty policy

You should submit all your assessment material via eLearning unless otherwise advised. The use of Turnitin for submitting assessment items has been instigated within unit sites. For more detail please see pages 3-5 the <u>BCA Student Assessment Guide</u>.

The BCA pays great attention to academic honesty procedures. Please be sure to familiarise yourself with these procedures and policies at your university of enrolment. Links to these and further information are available in the Welcome module in PSI and the <u>BCA Student Assessment Guide</u>. When submitting assessments you will need to indicate your compliance with the plagiarism guidelines and policy at your university of enrolment before making the submission.

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 students posting questions or accessing the solutions, and such students will be referred to and face disciplinary processes at their home university.

Late submission of assessments 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 is not able to approve extensions beyond three days; for extensions beyond three days you need to 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/978148222234

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
- 2. Azzalini A. *Statistical Inference: Based on the Likelihood*. Chapman and Hall, 1996. Modules 1 4.

- 3. Clayton D and Hills M. *Statistical Models in Epidemiology*. Oxford University Press, 1993. **Modules 1 4.**
- Casella G. Berger RL. Statistical Inference. Wadsworth and Brooks/Cole, 1990. Modules 1 – 4.
- 5. Gelman A, Carlin JB, Stern HS, Dunson DB, Vehtari A and Rubin DB. *Bayesian Data Analysis (3rd ed.)*. Chapman and Hall/ CRC Press, 2013. **Module 5.**
- 6. Lee PM. Bayesian Statistics. Oxford University Press, 1989. Module 5.
- 7. Mood, A.M., Graybill, F.A. & Boes, D.C. (1963). *Introduction to the theory of statistics* (*3rd ed.*). McGraw-Hill. **Modules 1 4.**
- 8. Wackerley, D., Mendenhall, W., & Schaeffer RL. (2008). *Mathematical Statistics with Applications*. Wadsworth Group. **Modules 1 4.**

Many statistical textbooks are not entirely devoted to inference, but have several sections on inference, which may not be as theoretical as the books above, such as:

- Altman DG. Practical Statistics for Medical Research. Chapman and Hall, 1991
- Fisher LD, van Belle G. *Biostatistics A Methodology for the Health Sciences.* Wiley, 1993.

Software requirements and assumed knowledge

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. The recommended and supported software for this unit is Stata and R. Whenever you will be required to use statistical software, the necessary code will be downloadable from the PSI eLearning site. The code can be run on your computer, and usually will only need to change input values for exercises / assignments.

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.

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 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.

Changes to PSI since last delivery

(including changes in response to student evaluation)

From Semester 1 2022 there are 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 the PDT unit and thus will be revision for many students. Other PSI modules have also been rearranged.

PSI is delivered in both Semester 1 and Semester 2 each year. 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 will be providing the opportunity for live consultation (either in the form of tutorial or Q&A sessions, depending on demand) 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
- A/Prof Patrick Kelly
- Prof Ian Marschner
- Justin Zeltzer