Biostatistical tools for clinical research

Contents

1 Introduction

This subject will cover the basics of Epidemiology and Biostatistics with a view of providing tools that clinical researchers require to design, conduct and disseminate medical and health research.

This unit covers topics associated with both the design of, and analysis of data arising from clinical research projects. It covers the full range of study designs, and focuses on the statistical implications of study design, including strategies to deal with designs more susceptible to bias. Participant is strongly encouraged to progress through the content based on the structure provided on page 3. It is important to note that biostatistics is inherently conceptual and can involve abstract mathematical ideas, and as such, understanding later content requires mastering earlier content. In other words, the student is strongly encouraged to keep up with content as the subject progresses.

2 Rationale

Evidence-based medicine is built on a framework of logical, systematic, replicable, and most importantly, defensible steps that allow the reader of the research (including reviewers and examiners) to gauge the strength of evidence of the work. Without a firm understanding of research methodology, those who attempt to conduct such research will be unable to produce quality research. In contrast, those who do have a firm grasp of research methods (including epidemiology and biostatistics) are well placed to publish their research in leading journals, leading to work of greater impact, and in turn, to changes in policy and/or clinical practice, and leadership in their clinical field.

This subject will cover three important and linked aspects of quantitative clinical research:

1. **Clinical epidemiology** (particularly that concerned with study design and it's link to quality of evidence);
2. **Statistical models** appropriate for dealing with most of the different types of clinical outcomes and designs that arise in medical research
3. **Statistical computing**: The tools available for implementing the above models, and answer research questions

3 Aims

The aim of this subject is to familiarize the participant with the concepts underlying, and tools employed to conduct, quantitative clinical research.

4 Objectives

At the end of this subject, the student should be able to:

1. Understand study design and it's implications for strength of evidence and choice of biostatistical approach;
2. Understand the standard linear models commonly used in clinical research, and appreciate their relative strengths and limitations; and
3. Conduct analyses based on the models in (2) and disseminate their results

5 Teaching approach

The concepts covered in this subject will be delivered using two formats: Lectures and hands-on workshops. The proposed content and it's sequence of delivery is provided in the following section. Data arising from real Thai studies will be used to reinforce the methods introduced.

There is no required text for this subject, although the students are strongly encouraged to use resources at their disposal to clarify many of the concepts and models covered. Specific resources will be identified for the participants throughout the course of the subject.

6 Content and structure

Week	Lecture	Workshop
Week 1	Clinical outcomes, study design and approriate statistics	
Week 2	(a) Hypothesis testing, estimation, CIs and p-values; (b) Introduction to statistical computing in R	Hands-on: Descrptive statistics and basic graphics in R
Week 3	Classical Biostatistical methods	Hands-on: Classical methods in R
Week 4	Correlation and Linear Regression	
Week 5	ANOVA and the General Linear Model	Hands-on: Modeling continuous outcomes in R
Week 6	The Generalized Linear Model and Binary Logistic Regression	
Week 7	Modeling polytomous outcomes	Hands-on: Logistic and Poisson regression in R
Week 8	Survival analysis: Modeling time-to-event outcomes	Hands-on: Survival analysis in R
Week 9	Longitudinal studies: Continuous outcomes	Hands-on: Linear Mixed Models (LMMs) in R
Week 10	Longitudinal studies: Categorical outcomes	Hands-on: Generalized estimating equations and Generalized LMMs in R
Week 11	Sample size	Hands-on examples including write-up
Week 12	Clinical biostatistics: (1) Gauging reliability of clincal measures	Hands-on: R examples
Week 13	Clinical biostatistics: (2) Evaluating diagnostic tests and	Hands-on: R examples

7 Assessment

Unlike content based subjects, biostatistics is a process based discipline. Devloping an understanding of biostatistical analysis is gained through 'doing'. There are two main objectives in this subject. The development of proficiency in introductory to advanced statistical methods (to the level of performing the analysis and understanding the corresponding results), and the ability to disseminate statistical approach used and the corresponding results. With this in mind, the assessment of this subject will emphasize the students ability to convey statistical methods and results, as communicated in a scientific monograph. Emphasis will be placed on the students ability to write a strong methods and results section.

This subject will involve two assessment items:

1. Assignment 1: Modelling continous and binary outcomes (50%) (handed out at the end of week 6)
2. Assignment 2: Modeling of time-to-event and longitudinal outcomes (50%)

Both of these assessment items are to be presented in a report format where the main components might represent the methods and results section of a scientific monograph. More details on the format will be provided at the time the assignment is handed out.