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## *Preface*

Health economics is a relatively new discipline, essentially characterised by the integration of different expertise and perspectives. Clearly, the clinical aspect is fundamental and the clinical background plays a basic role in the definition of any health economic evaluation. However, in its modern incarnation health economics is effectively identified by the integration of economic models and increasingly advanced statistical techniques, particularly under the Bayesian approach.

The objective of this book is to give a specialised presentation of these techniques. The book is thought as a manual for an advanced course in statistical methods for health economics and assumes some knowledge of statistics. Specifically, throughout the book we develop models and examples using a combination of R and WinBUGS/JAGS for the main Bayesian analysis (usually based on Markov Chain Monte Carlo).

The structure of the book is the following. In chapter 1, which is written in collaboration with Rachael M. Hunter, we introduce the main economic concepts; among them, the important distinction between financial and economic analysis and the definition of the relevant costs for the health economic evaluations. The typical outcomes are also discussed, with particular reference to the distinction between “hard” clinical outcome and measures of utility derived for example by suitable questionnaires. Then, we move to discuss the main types of economic evaluations, focussing our attention particularly on cost-effectiveness and cost-utility analysis. Finally, we discuss basic health economic concepts such as the *Incremental Cost-Effectiveness Ratio* and its use as a measure of comparison among different interventions. Some simple examples are presented to clarify the computational aspects and the interpretation of the results.

Chapter 2 presents the fundamentals of Bayesian statistics. Particularly in this chapter, some working knowledge of statistics is assumed. First, we briefly revisit the main characteristics of the Bayesian philosophy and the meaning of subjective probability. The concept of rational decision-making in the face of uncertainty (which will be considered also in chapter 3) is discussed before moving to parametric models. Exchangeability in its implications in the Bayesian paradigm is also touched upon before moving on to the main inferential aspects. The choice of the prior distribution and its combination with the available data into the posterior distribution are presented through a set of examples. Finally, we discuss simulation techniques (such as Monte Carlo and Markov Chain Monte Carlo) in simple terms. Although the methods

are discussed in details, with the aim of guiding the unfamiliar reader through their potential and limitations, the mathematics is kept at a relatively low level.

Chapter 3 first reviews the basic concepts underpinning the application of Bayesian decision theory. In particular, we stress the importance of the decision criterion based on the maximisation of expected utilities as an equivalent means of maximising the probability of the outcome preferred by the decision-maker. We do so briefly and keeping a very low level of the mathematics involved. Then we specialise the framework to the specific health economic evaluation. This has additional complications, *e.g.* the fact that the utility function needs to be specified over two relevant dimensions (costs and clinical benefits). Thus specific methods for the application of Bayesian health economic evaluation is developed. In doing this, we continuously switch between the theoretical aspects and a practical application to a relatively simple, fictional problem. Finally, we move on to define and discuss the theory and practice of probabilistic sensitivity analysis (PSA), *i.e.* the quantification of the impact on the decision analysis of the underlying uncertainty in the model. We consider both PSA with respect to parameter and structural uncertainty.

After having discussed in the two previous chapters much of the required theory, we then move to give a detailed account of “how to do” Bayesian analysis (in chapter 4) and health economic evaluation (in chapter 5). In chapter 4, we concentrate the attention to the computational aspects of Bayesian inference and we develop a series of worked examples combining the use of R (for pre- and post-processing of the data and the results) and JAGS to execute the Bayesian estimation.

Finally, chapter 5 presents a few worked examples of health economic evaluations, which we develop using the tools discussed in the previous chapters. We present some of the problems typically encountered in the analysis of health economic data: the first example considers a case in which individual data are available on both cost and clinical benefit data and we use several possible distributional assumptions to perform the cost-effectiveness analysis. In the second case, we first discuss the theoretical aspects of Bayesian hierarchical models and their use in the development of evidence synthesis models, which are particularly useful in health economics. Once a suitable decision model is specified, the relevant probability distributions to be associated with the random quantities are defined starting from empirical data retrieved from the literature, rather than from observations on individuals. Finally, the third case shows an application of Markov models, an increasingly popular methodology that is particularly effective in representing dynamically the progression of patients through a set of clinically relevant states. We show two different examples, one in which the main components of the model (*i.e.* the transition probabilities) are directly estimated using observed data; and the other in which they are defined as functions of suitable parameters, which are the objective of the Bayesian estimation procedure.

The final version of the book has benefitted from comments and sugges-

tions by many friends and colleagues. Among them, I would like to mention Christian Hennig, Gareth Ambler, Marta Blangiardo, Giampiero Marra and Maurizio Filippone. Martyn Plummer, Chris Jackson and two anonymous reviewers provided me with some insightful comments, particularly on chapter 4.

Philip Dawid has been instrumental in helping me formalise the material in chapter 3, while Rachael Hunter has brought a much needed economist's perspective to chapter 1.

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Gianni Corrao and the students of the course *Statistics for health economics* which I taught in the University of Milano Bicocca (Italy) have been exposed to a previous version of the material presented in the book and have helped me find a balance in the topics.

Robert Calver and Rachel Holt have been the best people I have ever worked with at Chapman Hall — OK: they are also the only ones I have ever worked with at Chapman Hall, but that does not take anything away from their brilliant work and continuous support!

Finally, like a proper good Italian, I should also thank my *mamma* and *babbo*. If readers shared just a fraction of their unconditional love and excitement for this book, it would change the landscape of health economics for ever. Of course, I do hope that readers will have more interest in the actual contents of the book and not just in the fact that my name is on it!

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