Statistical Analysis of Cost-effectiveness Data

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Statistical Analysis of Cost-effectiveness Data
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For Bernie

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to the Emma and Lucy O’Brien Education Fund.
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Preface

This book describes statistical methods applied to cost-effectiveness analysis. It represents the experience over many years of the author’s involvement in the application and methodology of health economic evaluation. The focus on randomised clinical trials reflects the fact that the trend towards collecting not only clinical, but also economic, data alongside clinical trials was the driving force behind many of the methodological developments described in the text. Health economics is a relatively young discipline and the use of clinical trials as a vehicle for economic evaluations began in earnest only twenty years ago. As a consequence, there has been a high degree of methodological development since then, with most of the reporting confined to journal articles. The aim of this book is to draw together those developments in a single source which we hope will be of interest to students of statistics, keen to understand more about health economics, and students of health economics, keen to understand the statistical methods required for undertaking economic evaluation of health care interventions. The exposition is at a technical level roughly equivalent to that found in final year undergraduate mathematics and statistics courses or postgraduate social sciences courses.

The book itself naturally divides into two parts. The first part (up to Chapter 5) deals with the established approach for the presentation of cost-effectiveness analyses, with a focus on estimating health outcomes and resource use costs. The second part of the book (Chapters 6 through 9) handles specific issues in more depth to give a fuller understanding of the nuances of a modern cost-effectiveness analysis where patient-level data are available.
In the preparation of any book there are numerous colleagues and students who have provided the inspiration and insight, as well as friends and family who have provided the encouragement and support, necessary to bring such a project to fruition. We are extremely grateful to all those people who have helped us over the years and aided us to a greater or lesser extent in supporting our endeavours and correcting our mistakes. However, one person stands out as the true inspiration for this book. A friend and colleague who had a major influence on both of our careers in the area of health economic evaluation, albeit in different ways, Bernie O’Brien was a rare person – someone with a keen intellect, an infectious enthusiasm, and a generosity of ideas that could not fail to rub off on those around him. His untimely death on the 13th of February, 2004 was a terrible shock and leaves a vacuum in the health economics community, as well as for his wife Karen and daughters, Emma and Lucy. We dedicate this book to Bernie’s memory.
1 Concepts

1.1 INTRODUCTION

There is a growing expectation from health care policymakers that evidence supporting the cost-effectiveness of new health care interventions, particularly pharmaceuticals, be provided along with the customary data on efficacy and safety. In Australia (Commonwealth of Australia, 1990) and Canada (Detsky, 1993) there are formal requirements that pharmaceutical companies present evidence of cost-effectiveness before a drug is granted reimbursement status on a formulary. In the United States there is demand for such economic data from third-party insurers, see Leaf (1989).

There are two general approaches to performing an economic evaluation of a health care intervention, see O’Brien (1996). One approach combines the efficacy and safety data from randomized clinical trials (RCTs) with cost data from secondary, non-trial sources in a decision analysis model. In such models the problem of inferential uncertainty is addressed using sensitivity analyses to determine what effect varying the model assumptions has on the results, see Briggs et al. (1994). The other approach uses health care utilization data collected on individual patients prospectively as part of an RCT. The health care utilization data combined with the appropriate price weights yield a measure of cost for each patient. Measuring effectiveness and cost at the patient level permits the use of more conventional methods of statistical inference to quantify the uncertainty due to sampling and measurement error. Since the early 1990s, when such data became