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Conclusions and Implications for Future Research

10.1 INTRODUCTION

This book has described the general use of Bayesian methods in evaluation of health-care interventions, and has considered a number of specific areas of application. Whilst in many of these areas the advantages of adopting a Bayesian approach appear clear, a number of problems have also been identified. Section 10.2 summarises many of these advantages and disadvantages. Section 10.3 identifies areas requiring further research and makes a series of recommendations for the main participant groups in health-care evaluation. These conclusions are deliberately expressed in a 'list' style.

10.2 GENERAL ADVANTAGES AND PROBLEMS OF A BAYESIAN APPROACH

Potential advantages of Bayesian approaches in health-care evaulation

1. All evidence can potentially be taken into account.
2. Specification of a prior distribution requires sponsors, investigators and policy-makers to think carefully and be explicit about what external evidence and judgement they should include.
3. Hierarchical models, which also can be handled within a non-Bayesian framework, allow pooling of evidence and 'borrowing of strength' between multiple substudies.

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4. Potential biases can be explicitly modelled, allowing the synthesis of studies of varying designs.
5. The Bayesian approach focuses on the vital question: how should this piece of evidence change what we currently believe?
6. Probability statements can be made directly regarding quantities of interest, and predictive statements are easily derived.
7. Juxtaposition of current belief with clinical demands provide an intuitive and flexible mechanism for monitoring and reporting studies.
8. The inferential outputs from a Bayesian analysis feed naturally into a decision-theoretic and policy-making context.
9. Explicit recognition of the importance of context makes Bayesian methods particularly suitable for evaluation of health-care interventions, in which multiple parties may well interpret the same evidence in different ways.

Generic problems

1. Unfamiliarity with Bayesian techniques, perhaps along with their perceived mathematical complexity, and some conservatism on the part of potential users, has resulted in limited use of proper Bayesian methods to date.
2. The use of prior opinions acknowledges a subjective input into analyses, which may appear to contravene the scientific aim of objectivity.
3. Specification of priors, whether by elicitation or choice of defaults, is a contentious and difficult issue.
4. There are no established standards for design, analysis and reporting of Bayesian studies.
5. There is a danger that the additional complexity of Bayesian methods will lead to poor use.
6. A full decision-theoretic framework can lead to innovative but non-standard trial designs which may be very different from those currently in use.
7. Specification of expected utilities is difficult and may require extensive assumptions about future use of interventions.
8. Computational complexity of the methods has until recently been a major issue.
9. Software for implementation of the methods is still limited in availability and user-friendliness.

10.3 FUTURE RESEARCH AND DEVELOPMENT

We have claimed that Bayesian methods could be of great value when evaluating health-care interventions. For a realistic appraisal of the methodology, it is useful to distinguish the roles and requirements for six main participant groups: methodological researchers, sponsors, investigators, reviewers, policy-makers and consumers (see Sections 3.1 and 9.2). However, two common themes for all

participants can immediately be identified. The first is the need for an extended set of case studies showing practical aspects of the Bayesian approach, in particular for prediction and handling multiple sub-studies, in which mathematical details are minimised but details of implementation are provided. We hope the examples in this book have contributed towards this goal. The second theme is the development of standards for the performance and reporting of Bayesian analyses, possibly derived from the checklist described in Section 3.21 and used throughout this book.

1. *Methodological researchers*. With regard to design, there is a need for transferable methods for sample-size calculation that are not based on Type I and Type II error, such as targeting precision, and realistic development of payback models, including modelling of dissemination. Simple and reliable elicitation methods for the priors of 'non-enthusiasts' require testing, as well as demonstrations of the use of empirical data as a basis for prior distributions. Reasonable default priors in non-standard situations need to be available. Methods for flexible model selection and robust MCMC analysis require development and dissemination, and there is a need for user-friendly software for clinical trials and evidence synthesis.

   It is essential to have appraisal criteria along the lines of the checklist used in this book, with possible reformulation as guidelines along the lines of 'How to read a Bayesian study' – it would also be useful to have the term 'Bayesian' in all relevant papers in order to aid literature searches. Finally, increased integration with a health-economic and policy perspective is highly desirable, together with flexible tools for implementation.
2. *Sponsors and investigators*. Both public sector and industry could extend their perspective beyond the classical Neyman–Pearson criteria, and in particular investigate quantitative payback models. The pharmaceutical industry might also investigate formal project prioritisation schemes. All sponsors could focus on the evidential basis for assumptions made concerning alternative hypotheses and the potential gains from technology, and use empirical reviews to establish reasonable prior opinions. There is also potential for 'open' studies in which interim results are reported to investigators.

   It would be valuable to gain experience in eliciting prior opinions from both enthusiasts and a general cross-section of the target community. There is great scope, when analysing data, to go beyond the usual limited list of models and consider a range of priors and structural assumptions. Finally, when reporting a study, it is vital that any Bayesian reporting allows future users to include the evidence in their synthesis or decision. The use of our checklist or a similar scheme for reporting should help in this.
3. *Reviewers/regulatory bodies*. Regulatory bodies could establish reasonable prior opinions based on past experience in order to provide default priors, and could take a more flexible approach to the use of data,

particularly in areas such as medical devices, and encourage efficient use of data by appropriate use of historical controls, evidence synthesis and so on. More experimental would be the explicit modelling of the consequences of decisions in order to decide evidential criteria.

4. *Policy-makers*. There is a need for careful case studies in which policy-makers explicitly go through the following stages in reaching a conclusion based on a full Bayesian analysis:
   - *Priors*. Specify prior opinions relevant at the time of decision-making.
   - *Modelling*. Pool all available evidence into a coherent model.
   - *Reporting*. Make predictive probability statements about the consequences of different policies.
   - *Decision-making*. Assign costs to potential consequences, and so assess (with sensitivity analysis) the expected value of different actions.
5. *Consumers*. Clinicians might be expected to exercise their subjective judgement concerning how their own prior beliefs are influenced by available evidence, while individual patients' utilities values can be elicited to see, for example, whether a population-based decision made by a health-care agency matches one based on their personal opinions.