

CHAPTER 1

From effectiveness to efficiency? An introduction to evidence-based decisions and economics

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'The question we ask today is not whether our government is too big or too small, but whether it works - whether it helps families find jobs at a decent wage, care they can afford, a retirement that is dignified. Where the answer is yes, we intend to move forward. Where the answer is no, programs will end. And those of us who manage the public's dollars will be held to account - to spend wisely, reform bad habits, and do our business in the light of day - because only then can we restore the vital trust between a people and their government.'

(President Barack Obama, Inaugural Address, 20th January 2009)¹

Introduction

'Evidence-based policy and practice' is a phrase commonly used to refer to public policy and professional practice informed by the application of rigorous methods to the search and review of evidence – a process often described as a 'systematic review of evidence'.

Although previous methodologists had already advocated the use of experimental evidence and synthesis of this evidence for practice decisions in many

fields, the term 'evidence-based decision making' achieved widespread use in the field of medical policy and practice.^{2,3} The epidemiologist Archie Cochrane, who challenged the medical profession to base their practice on evidence from randomised controlled trials (RCTs), stimulated this.⁴

Inspired by Archie Cochrane, the Cochrane Collaboration was established to prepare, publish and regularly update systematic reviews of the effects of health care interventions.⁵ Cochrane reviews have been available since the early 1990s.⁶ More recently, similar reviews of interventions in social welfare, education and criminal justice are appearing as a result of the work of the Campbell Collaboration, which is named in honour of DT Campbell, an evaluation methodologist with a particular interest in the value of experimental approaches to understanding the effects of interventions.^{2,7} Like the Cochrane Collaboration, the Campbell Collaboration publishes its systematic reviews electronically.⁸ However, advocacy for providing a summary of comprehensively sourced, replicable and quality-filtered information for trustworthy policy decisions, and development of methodologies for doing this, has a longer history, and this work underpinned the foundation of the Cochrane Collaboration and the Campbell Collaboration.^{3,9–11}

Methods for systematic reviews of evidence have concentrated mainly on the question of 'What works?'

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or 'Is this intervention effective in achieving a specific outcome?'. The questions 'Is it worth it?', 'At what cost is the outcome achieved?' and 'What will be the economic impact of this intervention?' have not been emphasised as much in the meta-analysis and systematic review literature to date. However, the concept and methods of economic and other forms of evaluation of the health and social impacts of interventions are well established and such approaches are being applied in evaluation of public sector projects across a range of policy areas.^{12–14} In an attempt to standardise approaches to the evaluation of public projects, governments have published methodological guidelines on the use of economic evaluation.^{15–24}

As the opening quotation to this chapter suggests, the need for evidence-based decisions that take account of both efficiency and fairness in society has received new emphasis as we prepared for this book. The challenge to researchers is not only to offer the tools for these decisions, but that the methods we advocate also meet President Obama's acid test: that they are efficient, effective, transparent and fair.

Evidence-based policy 'helps people make well-informed decisions about policies, programmes and projects by putting the best available evidence from research at the heart of policy development and implementation.'^{25–27} However, the ultimate purpose of evidence-based policy and practice could be rephrased as being to 'optimise the configuration and delivery of services in order to maximise individual and public welfare'. Economics has been defined as the study of the optimal allocation of limited resources for the production of benefit to society.²⁸ Decisions based only on highly focused evidence-based methodologies that consider only one dimension of the economic decision (i.e. whether the intervention works) may contribute to inefficient policy and practice and greater inequalities through lack of consideration of these potential trade-offs.^{29,30}

At the same time, a decision based on an economic study that does not utilise the most reliable evidence of effectiveness will also be flawed. In the field of health care in the UK, Archie Cochrane and Alan Williams were two influential leaders of their disciplines who acted as advisors to the Department of Health at the same time. Their own accounts of events illustrate how they agreed on the need to incorporate economic viewpoints, and how their work paved the way for the

National Health Service (NHS) to embed both health economics and evidence-based decision making into research and policy making in the UK.^{4,30,31}

In spite of the recent increase in recognition of the important role of economics in decisions across broader areas of policy and professional practice, as well as in the health care field, aspects of both systematic review and economic analysis make it difficult to neatly combine these approaches. This book is intended for those involved in decision making, whether your role is to provide evidence to support decisions or to use evidence to make decisions. Therefore, we do not assume depth of knowledge of either economics or systematic review. To set the scene for the book, the remainder of this chapter introduces some key concepts of systematic review and of economics. The final section introduces the structure and the contents of the book.

A brief introduction to systematic review

Systematic reviews attempt to collate all empirical evidence that fits prespecified eligibility criteria in order to answer specific research questions. They involve the use of explicit, systematic and replicable methods to assemble, select, critique and synthesise reliable and up-to-date evidence, from published and unpublished sources, on the effects of interventions.

The main purpose of systematic reviews of interventions has been to provide reliable, up-to-date evidence about the beneficial and harmful effects (outcomes) of interventions when compared to an alternative practice or form of care.^{3,32,33} The need for such an approach was advocated both by Archie Cochrane for the health field in 1972 and by DT Campbell for the field of educational psychology in 1963.^{2,4,34} The purpose was to overcome disagreements among experts on best practice that arise from reliance on unsubstantiated expert opinion, single studies, and incomplete or unsystematic reviews.

These views were further supported by methodologists such as Light & Pillemer, who elegantly illustrated the biases that can be introduced in attempts to sum up evidence for policy decisions, including examples on the educational debate on the effect of class size on students' performance, where misleading conclusions can be gained by simply counting the number of studies with significant findings for or against smaller classes, for

example.¹⁰ Light & Pillemer also emphasised the importance of drawing on a range of sources of evidence, including both qualitative and quantitative research.¹⁰

Application of systematic review methods on a large scale was pioneered in the health field by the Cochrane Collaboration, which has been publishing and maintaining reviews of the effects of health care interventions since 1992, followed by the establishment of the Campbell Collaboration, in 1999, to do the same in the fields of social welfare, education and criminal justice. Both organisations explicitly recognise the importance of best evidence in economic decisions. Neither organisation currently requires that its reviews necessarily include economic questions or methods; however, the fact that authors of reviews wish to do so is recognised by the inclusion of economics methods guidance in methods handbooks for reviews published by each Collaboration.^{35,36} The need to further develop guidance to assist authors and editors to make their reviews more useful for policy decision makers, and for economists to understand and engage in this process, is one of the principal motivations for development of this book.

In this book, a central focus is on systematic review as defined by the Cochrane Collaboration and the Campbell Collaboration, in which a protocol is developed and published that describes questions and methods to be used. Then, as laid out in the protocol, a comprehensive search for evidence is conducted, also seeking relevant unpublished findings. In this process a ‘map’ of research evidence that is known to have been published to address a specific question, and what is missing, is formed. When there is enough research evidence that meets preset criteria for inclusion, the evidence is synthesised from a subset of sources that minimise the risk of bias in the comparison of options. This can include meta-analysis of critically assessed RCTs and other types of robust comparative study.

A brief introduction to economics for the non-economist

Resources, such as people’s time and skills, raw materials, land and energy, are needed as the inputs for providing goods and services. Economics is about how resources are used to meet needs and wants at different levels, including the individual, the household or the state. The efficient economy has been

defined as one where no change in resource use can make anyone better off without making someone else worse off. This principle was first proposed by the economist Vilfredo Pareto (1848–1923). It works up to a point where there is spare capacity in the system, but in a busy, already ‘Pareto-efficient’ economy, trade-offs need to be made when new policies are introduced. That is, if all resources are fully used, some existing production must be changed, and output lost, with an associated loss of benefits from that use. This loss is known to economists as ‘opportunity cost’. Judgements about costs and benefits are then needed between stakeholders who ‘win’ (benefits) and those who ‘lose’ (costs).

Where resources are bought and sold, there is a price for each unit of resource (such as staff salaries, rent for buildings, the price of computers, books, medicines, etc.). Money, which is used as an indication of value and a medium of exchange, is an important part of most economies, oiling the wheels of exchange of goods and services. Economic theory, based on a strict set of assumptions, suggests that unregulated economic markets can provide an optimal allocation of resources. In practice, these assumptions are not met, and most markets for goods and services fail to clear themselves efficiently or equitably for all those involved.

Markets are rarely (many would say never) ‘perfect’ for many reasons. There is lack of information to both producers and consumers about the nature and quality of goods and services and about consumer needs and wishes, both now and in the future. Some buyers and sellers in the market can individually determine price levels through monopoly power. Other factors cause barriers to market entry by producers, such as trade association or professional agreements, or the viable size of production units needing prohibitively large investment or a lack of mobility of resources. Consumers of services are also constrained in how they participate in the market by their personal resources (e.g. income, support networks and ability) and by whether there are any alternatives to the good or service they are seeking. In addition, there are some goods and services, known as ‘public goods’, that are seldom regarded by entrepreneurs as worth producing and selling in the free market because, once they exist, they benefit everyone whether they have paid or not. Examples of public goods include public sanitation and crime prevention initiatives.

Because markets fail in areas of social and political importance to individual and public welfare, such as health, social welfare, education and criminal justice, governments intervene in different ways and degrees around the world. Services and funding are provided through publicly regulated organisations in various ways. Although it is unusual in most economies for state provision to be managed at all levels with no element of market activity or private sector involvement, it is also recognised at government level that other 'non-market' approaches for optimising economic welfare are needed to aid decision makers choosing policy and guiding practice in public services. For this reason, among others, both economic analysis and evidence-based approaches are used.

Guidance on methods to inform policy and practice decisions

How should we go about compiling the best evidence on the economics of interventions in addition to their effectiveness? Can the systematic review approach aid reviews of broader types and bodies of evidence, including economic evidence? Can economic evaluation and other forms of economic analysis be conducted systematically and transparently? What guidance do we have and what are the strengths and limitations of different approaches?

Decisions on policy and practice take place at many levels, from individual practitioners and service users to governments. Although groups informing consumers and professional and regulatory bodies do not all take the same approach to decision making, they are all interested in the best, least biased evidence and in optimal use of resources. Around the world, governments have published guidance to evaluators so that the evidence used to inform decisions is based on comparable methods. For example, in the United States, the Office of Technology Appraisal published guidance between 1975 and 1995 that has been used as the basis for many international variants on guidance on technology appraisal in different countries, especially in the health care field.^{15–18} This guidance includes emphasis on the need for systematic reviews of effects and economic evaluation. In the UK (as in several other countries), the Treasury publishes a handbook for evaluators of public

projects, which includes reference to both quality of evidence and the approach to be taken for economic evaluation.¹⁹

Further review of the guidance on evaluation across policy areas highlights many areas of difference, all of which pose important questions for those who, like the authors and editors of this book, are engaged in efforts to guide the systematic review community in providing economic evidence relevant to decisions about interventions implemented in more than one policy domain and in (or, in some cases, across) different economic systems, which takes account of the distribution of costs and benefits within (or across) populations.^{21–23}

Overview of the book

This book is about how the activities and outputs of evidence synthesis, systematic review, economic analysis and decision making interact within and across spheres of health and social policy and practice. It is also about the challenges that arise from these interacting processes.

In 2001, a group of economists and reviewers took part in a workshop in Banff, Alberta, funded by the Alberta Heritage Foundation, which led to the publication of the book *Evidence-based Health Economics* by BMJ Books in 2002.³⁷ This new volume, an entirely new edition, was prepared following a similar workshop at the London School of Economics and Political Science in November 2008. It follows the progress of the issues raised in the previous book and also takes a broader perspective, reflecting the importance of evaluation methods and decisionmaking beyond the health sector.

This book aims to:

- describe the current state of the art in approaches to evidence synthesis that combine economics and systematic review methods within and across the health care, social welfare, education and criminal justice fields
- examine the case for evidence-based principles in economic analysis, and the need for such principles to include an economic dimension.

The next three chapters build on this introduction to provide further insights into core methodological concepts and principles (Chapters 2 and 3) and the use of economic evidence in decision making (Chapter 4).

Chapters 2 and 3 take alternative perspectives. In Chapter 2, the focus is on how evidence is assembled and synthesised in decision models for economic evaluation, whilst Chapter 3 considers how economic perspectives and evidence drawn from previous studies can be assembled and synthesised in evidence reviews for policy decisions. Both chapters highlight questions of where economic thinking fits into evidence gathering and synthesis processes, and how systematic review methods relate to economic analysis methods. Chapter 4 builds on the preceding two chapters by providing an overview of how outputs from decision models and economic evidence reviews are currently used in the formulation of public policy and practice in different jurisdictions. The way in which policy makers and practitioners use information for decision making determines whether the work of evaluators and reviewers is likely to influence policy, practice and the delivery of services.

The next chapters cover a range of specific methodological issues facing evidence reviewers and economists. Two overarching concerns often expressed about systematic reviews and economic analyses are that their results may not be generalisable or transferable to other settings, and that they fail to consider the fair distribution of benefits and costs. Chapter 5 considers how an internationally used review, such as a Cochrane or Campbell review, might be relevant in different specific contexts, taking account of the complexity arising from the range of variables that can influence the final best answer. Chapter 6 illustrates and discusses how reviews and evaluations could, and should, go beyond the simple question of effectiveness and efficiency to consider equity.

Chapters 7–10 present developments covering several issues highlighted in the first edition of this book. Chapter 7 examines the evolution of literature search methods to identify evidence for systematic reviews and decision models that aim to support cost-effectiveness decisions. Chapter 8 addresses parallel issues in the identification and synthesis of health state utility values, which are commonly used to inform measures of the value of outcomes of health care interventions. Chapter 9 examines the use of evidence in decision models and how the use of different sources of evidence can influence model results. The authors propose hierarchies of sources of evidence to help limit the potential for bias in results.

The question of bias and quality is further addressed in Chapter 10, which presents frameworks for grading evidence on resource use, costs and cost-effectiveness and presenting these types of evidence flexibly and usefully for users of reviews.

Although it is common that health care and other systematic reviews place emphasis on the results of controlled trials of interventions, because this method is applied widely and provides least-biased evidence of treatment effects, it is also often the case that in the health field, as in other areas of social policy, the only and best evidence available is from observational datasets, such as those collected for administrative purposes or government surveys. Whilst the statistical and econometric methods used to analyse these data to detect the effects and costs of practices are unfamiliar to many analysts, meta-regression and other techniques are increasingly applied to help sum up findings in evidence reviews. Chapter 11 illustrates how meta-regression analysis can be used to explore the findings of studies, whatever methodology they employ.

A systematic review may find little or no relevant evidence, but this does not mean there is no policy decision to be made and, as such, analysis of evidence in reviews and economic evaluations can be used to estimate the value of new research. Chapter 12 profiles the use of evidence in value of information analysis to estimate the economic value of new research to answer policy questions.

Further criticism of the overall assumptions behind evidence-based approaches includes their failure to consider political priorities, and especially the impacts on inequalities in the distribution of benefits and access to services. Chapter 13 challenges the orthodoxy of assumptions underlying current approaches to evidence synthesis and presents the conceptual framework for a new approach.

Chapters 14 and 15 draw key implications of all the preceding material for current practice and future research. Chapter 14 summarises and discusses current recommendations for analysts utilising approaches to evidence synthesis that combine economics and systematic review methods. Chapter 15 presents a summary and discussion of priorities for further empirical research needed to develop the evidence base that underpins current and future research practice. Finally, to assist the reader, Chapter 16 provides a glossary of selected key terms used throughout the book.

Summary and invitation

The theoretical case for wanting to integrate economic analysis and systematic reviews is strong. In some cases, such integrated analysis can prove relatively straightforward and provide important results. However, the conduct of economic evaluation and other forms of economic analysis is not always straightforward. It will be seen in this book that different approaches to the same underlying issue can lead to different results, that there are significant negative consequences to not utilising the best evidence available at the time an economic analysis is conducted, and that there is not always a consensus on the best way forward.

In preparing this book, the editors and authors were aware that the first edition was firmly based in the health care field but that many of the policy issues faced in other related fields, including social welfare, education and criminal justice, are similar. Some of the authors and editors of this book are already working in these applied fields, and we have sought examples of applications from these fields. Our success or failure in this is reflected in the content of this book, and we are aware that there are areas that have less coverage than we hoped. Two specific areas we have identified are: methods for and examples of economic analysis in the field of education, and discussion of the role of econometric studies in policy formulation. Our success or failure has probably reflected the limits of our own networks, however systematically we have searched, and we look forward to finding like-minded colleagues willing to participate in our future work as a result of this book.

It is important that systematic reviewers and economists alike are aware of such issues, and that there is no single best way of integrating economic analysis and systematic review methods. In addition, it is necessary to think about the limitations of both economic analysis and systematic review more generally and, also, of the limits of integrating the two methodologies. If you are interested in some or all of the above, we invite you to join us on a journey 'from effectiveness to efficiency' and enjoy reading this book.

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