THE UNIVERSITY of York
CENTRE FOR HEALTH ECONOMICS

Making Decisions about Health Technologies: *A Cost-Effectiveness Perspective*

Frans Rutten
Michael Drummond

iMTA
institute for Medical Technology Assessment

MAKING DECISIONS ABOUT HEALTH TECHNOLOGIES:

A COST-EFFECTIVENESS PERSPECTIVE

by

Frans Rutten
Professor of Health Economics
and
Director of the Institute for Medical Technology Assessment
Erasmus University Rotterdam
Rotterdam, the Netherlands

Michael Drummond
Professor of Economics
Centre for Health Economics
University of York
York, United Kingdom

January 1994

Acknowledgements

This handbook for decision makers was produced as part of the EC Concerted Action on the Methodology of Economic Appraisal of Health Technology. We are grateful to the EC for financial support and to members of the Project Management Group for guidance.

Although we are sole authors of the handbook, we have drawn extensively on the original work of Martin Buxton, Linda Davies, Ben van Hout, Martin van Ineveld and Harry de Koning. Their contribution is gratefully acknowledged. Finally, we are grateful to Vanessa Windass and Caroline Verboom for secretarial assistance.

Contents

1. INTRODUCTION

The resources available to produce the goods and services that society values are scarce. There will always be more worthwhile objectives to pursue than there are resources to satisfy these objectives. The allocation of resources to a particular health care programme implies that these resources can not be used elsewhere; in other words, there are *opportunity costs* associated with a choice to invest in a health care programme. Economic evaluation is intended to assist us in choosing between alternative strategies for the allocation of resources and aims at systematically comparing the benefits and opportunity costs of each alternative strategy.

This handbook is intended to inform policy makers at various levels of decision making in health care about the technique of economic appraisal and to help them appreciate the potential roles of economic appraisal in health policy. In this context "policy makers" may be broadly defined: **politicians**, who may want to assess the role of this technique in general health policy; **civil servants** at the central government level, who may want to give this technique its appropriate place in health care legislation and administration; **health care insurers**, who may want to use the technique in selecting efficient health care providers and programmes; **health care managers**, who may want to use it in support of internal resource allocation; **health care professionals**, who may consider it as one of the essential elements for the development of practice guidelines and protocols, and finally, **manufacturers** of medical equipment and pharmaceutical products, who may see it as an appropriate instrument to position their products in a competitive health care market. Since the interest in the role of economic appraisal will differ between these decision makers, we will consider health policy at different levels of the health care system, but focus on

(central) government regulation as the area where economic appraisal currently has the most prominent role.

Choices regarding the allocation of resources to health care programmes affect the diffusion of technologies, and in considering the options for controlling such diffusion one may usefully distinguish between regulation by directive and regulation by incentive [see Rutten and Haan, 1990]. The former can be seen as a direct way of interfering with resource allocation, while the latter concerns policies influencing the diffusion of technologies and treatments in an indirect way. Table 1.1 provides an overview of possible regulatory mechanisms that may be supported by economic appraisal. Of course, the type of health care system determines the relevant mix of policy instruments. The policy instruments also differ with respect to the extent to which economic appraisal may support, or actually is supporting, the use of each. For instance, a well-known example of a pre-market control is the procedure of drug registration, which requires the demonstration of efficacy and safety of a drug before it is allowed to enter the market. In most countries, economic appraisal does not play any role in the associated procedure of approval. On the other hand, with respect to policies on reimbursement from public insurance schemes we have recently observed that in Australia and Ontario (Canada) the pharmaceutical industry is required (or may shortly be required) to include evidence on the cost-effectiveness of their products in submissions to the government committees deciding on the reimbursement of pharmaceuticals. So in this case the role of economic appraisal is firmly incorporated in the legislation or subsequent administrative procedure.

Table 1.1: Options for Control of the Diffusion of Health Care Technologies

Regulation by directive (central/ regional government)	Regulation by incentive
pre-market controls for drugs and devices	reforming payment schemes for health care institutions (e.g. hospitals)
(conditional) exclusion from public reimbursement	budgetary reform within institutions
planning of specialist facilities or specific technologies	changing payment systems for health care providers
	cost-sharing arrangements
	encouraging competition in the health care system
	medical audit and utilization review systems

Source: adapted from Rutten and Haan [1990]

At a lower level of decision making, for instance within the health care institutions, there is a trend towards making heads of departments accountable for their decisions on resource allocation, which could require them to use information from economic appraisal studies to support their investment- and operational decisions. An interesting development in this area has been the experimentation with clinical budgeting. Economic appraisal could be used to develop a clinical plan for a department and the associated budget. The use of evidence from economic evaluation studies may rationalize the negotiations between budget holders and central management.

The structure of this book is as follows. In Chapter 2 a brief description will be given of economic appraisal methods, the issues that are being debated and the problems that have to be solved to strengthen further its position in health care decision making. Furthermore, its current

role in health policy will be described and some trends identified. In chapter 3 we argue that the mix of regulatory mechanisms and type of health system sets specific requirements for the economic appraisal study to be useful in each context. Depending on the type of decision for which economic data are required, there will be emphasis on different aspects of the economic appraisal. This is illustrated by the presentation of three case–studies in Chapters 4 – 6. Chapter 7 concludes by drawing some general lessons from the case–studies presented, and by giving recommendations on further strengthening the link between economic research and health policy.

In producing the handbook we have recognized that decision makers have different levels of prior knowledge about economic appraisal methods. In order to make the handbook accessible to all groups we decided to assume no prior knowledge. Therefore, those decision makers already having some background knowledge may prefer to skip Chapter 2 and go straight to the case studies, in which case Chapter 2 is always available for reference.

Reference

Rutten, F., Haan, G. Cost–effective use of medical technology: regulatory instruments and economic incentives. In: B. Jönsson, F. Rutten and J, Vang (eds.) Policy making in health care: changing goals and new tools. Linköping, Linköping Collaborating Centre, 1990.

2. THE ROLE OF ECONOMIC APPRAISAL

2.1 Introduction

Given the scarcity of resources for health care, there is a growing interest in economic appraisal. Economic appraisal of health care programmes and treatments have now been conducted for the least 30 years. The key methodological principles have been specified and a number of textbooks have been published [Drummond, 1980; Warner and Luce, 1982; Drummond et al., 1987; Luce and Elixhauser, 1990]. Also, over the past five years there has been an exponential rise in the number of published studies. These have assessed treatment alternatives in all branches of medicine.

However, much less has been written about the role of economic appraisal in developing policies for an efficient diffusion and use of health technologies. Therefore, this chapter discusses this issue in the following manner. First, a brief introduction is given to economic appraisal methods. Secondly, the main problems in methodology are identified. Thirdly, the policy issues (relating to health technology) that are amenable to economic appraisal are identified. Fourthly, the ways of increasing the relevance of economic appraisal results to health technology are discussed and, finally, a few conclusions are drawn.

2.2 Principles and Forms of Economic Appraisal

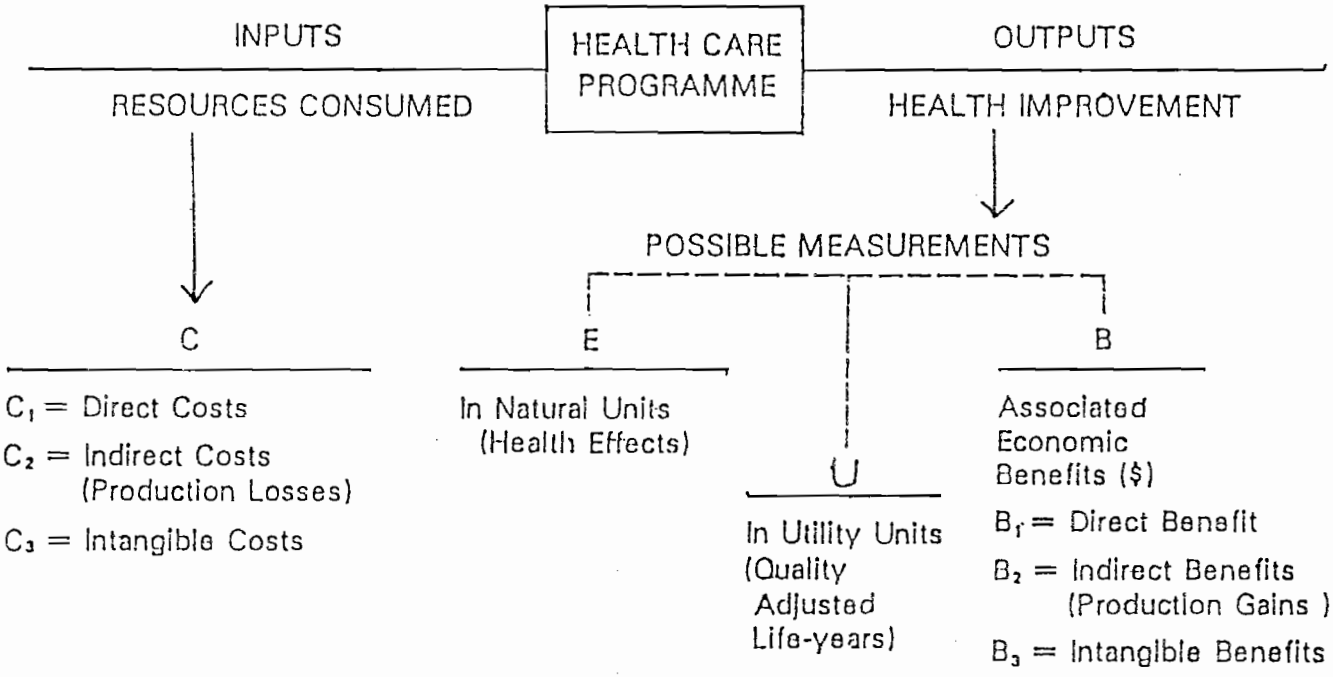
There are a number of forms of economic evaluation, but they have the common feature that some combination of the inputs to a health care programme are compared with some

combination of the outputs (Figure 2.1). The inputs include the direct costs of providing care (C1 in Figure 2.1), which fall mainly (though not exclusively) on the health care sector, and the indirect costs (in production losses) arising when individuals are withdrawn from the workforce to be given therapy (C2). Although not strictly an 'input', there may also be intangible costs, in pain or suffering, associated with therapy (C3).

The simplest form of analysis considers only costs. This approach is justified where it can be assumed, or has been previously shown, that the alternative programmes or therapies being compared produce equivalent medical results. This was the approach used by Lowson et al. [1981] in their study of alternative methods of providing long–term domiciliary oxygen therapy. Such a study is called a cost analysis, or cost–minimization analysis. Some cost analyses confine themselves to consideration of direct costs only, others consider also the indirect costs.

One particular form of cost analysis deserves further mention since it has had wide application. The cost of illness study calculates all the direct and indirect costs of a particular disease or illness, such as stroke or cancer [Hartunian et al., 1980]. These studies can serve two purposes, depending on how they are carried out. First, by providing an estimate of the economic impact of a given disease, they can alert policy makers to the importance of the problem and suggest that investments should be made in interventions to ameliorate its effect. Secondly, they can provide a baseline estimate of costs against which the potential economic impact of a new medicine can be judged.

Figure 2.1: Components of Economic Evaluation



However, most forms of economic evaluation require explicit measurement of the outputs of the programmes or therapies being compared. They differ mainly in the method of measuring the outputs. The earliest forms of analysis concentrated on the benefits of interventions in terms of the resulting savings in other direct medical care costs (direct benefits, B1), and the production gains from an earlier return to work (indirect benefits, B2). Typically, in a cost–benefit analysis, these benefits were expressed in money terms in order to make them commensurate with the costs of the intervention. However, other more intangible benefits, such as the value to patients of feeling healthier (B3), are obviously more difficult to express in money terms. Therefore cost–benefit analyses have often been criticised for ignoring important benefits from health care programmes and for concentrating on items that are easy to measure. Many of the early studies were therefore very narrow assessments, considering only direct and indirect costs and benefits. However, more recently there have been some good examples, such as the study by Weisbrod et al. [1980], which assessed a wide range of costs and benefits in a comparison of hospital–oriented and community–based care for mental illness patients. The authors were able to demonstrate that the community–based service had higher net benefits than the hospital–oriented alternative.

Instead of attempting to measure outputs in money terms, other analysts have preferred to assess them in the most convenient natural units (health effects), such as 'cases successfully treated' or 'years of life gained'. For example, Hull et al. [1981] compared objective diagnostic tests for deep–vein thrombosis in terms of their incremental cost per case detected, over and above normal clinical diagnosis. Ludbrook [1981] compared treatment options for chronic renal failure in terms of their cost per life–year gained. Such analyses are known as cost–effectiveness analyses.

Of course, many health technologies are concerned with improving the quality, not quantity, of life. In addition, some therapies, such as cancer chemotherapy or hypertension treatment, may bring about slight reductions in the quality of life in order to extend life. Therefore, there has been a growth in interest in cost-utility analysis, where the life-years gained from treatment are adjusted by a series of utility weights reflecting the relative values individuals place on different states of health [Drummond et al., 1987]. The output measure most frequently used in cost-utility analysis is known as the quality adjusted life year (QALY). An example of cost-utility analysis is the study by Boyle et al. [1983], who calculated the cost per quality-adjusted life-year gained from providing neonatal intensive care to very-low-birthweight infants.

In recent years there has been a rapid expansion in the publication of cost-benefit and cost-effectiveness analyses, particularly in medical journals, so that evaluations are now available for many choices in prevention, diagnosis, therapy, location of care and organization of services. In judging this literature the decision maker needs to answer two questions; 'is the study methodologically sound?' and 'does it apply to my setting?'. In order to help the decision maker resolve these issues a 10 point checklist of questions to ask about a published study has been developed and applied in the assessment of studies in chronic bronchitis, treatment of hypertension, neonatal intensive care, prevention of pulmonary embolism and community care for mental illness [Drummond et al., 1987].

As in all fields of scientific inquiry, it is important to be clear on the study question. In particular, the viewpoint(s) from which the alternatives are being compared should be clearly identified. Questions such as 'Is the new medicine worthwhile in the prevention of coronary heart

disease?' beg the questions 'to whom?' and 'compared to what?'. A better specified question would be something like the following: 'From the viewpoint(s) of (a) the Ministry of Health; (b) other agencies providing care and (c) patients and their families, would a preventive programme including a new medicine be preferable to the existing programme, which concentrates mainly on treating coronary heart disease as and when it occurs?'.

It is important that studies include a comprehensive description of the competing alternatives so that the decision maker can assess the implications of study results for his own setting. In the case of the evaluation of medicines it would be important to specify the dosage levels, the mode of administration, the length of treatment, and the extent of monitoring of the patient's condition that is required.

Of course, given the need to consider both the costs and consequences of interventions in an economic evaluation, it is important that the effectiveness of the programmes or treatments is established. This emphasizes the need to integrate the economic evaluation of health technologies as fully as possible with their clinical evaluation.

The identification, measurement and valuation of all relevant costs and consequences is also important. Obviously the range included needs to match the breadth of the viewpoint(s) being considered and the study question being posed. In particular, broader questions demand that a wider range of costs and benefits is measured and valued, since frequently the issue of whether the treatment is worthwhile, when compared to the alternative uses of the same resources, is being explored.

If the costs and consequences of the alternatives occur at different points in time they need to be adjusted for differential timing by discounting to present values [Drummond et al., 1987]. (This reflects the fact that events occurring in the future are viewed by individuals as being less important than current events.) Furthermore, sensitivity analysis should be performed, exploring the sensitivity of study conclusions to the values of those parameters about which there may be methodological controversy or imprecision in estimation. Typically, the factors varied in a sensitivity analysis include the discount rate, the costs of (or savings from reduced) hospitalization, the medical evidence on the success of therapy and the relative valuations of states of health. The precise selection of items for inclusion in a sensitivity analysis depends on particular circumstances, but users of evaluation results should be suspicious of a study that does not embody this general approach, as it is likely that many of the estimates used are more optimistic than would be found in practice.

Finally, in the presentation of results, it is normal to show an incremental analysis. That is, compared to the existing programme or treatment, what extra costs and extra benefits would result if the new technology were used? It should be remembered that where the implicit existing programme is 'doing nothing', this rarely results in zero costs and zero benefits. In addition, the presentation of results should include a discussion of other concerns to users, such as the implications for other policy objectives (e.g. equity), the managerial costs of changing to the recommended intervention and the extent to which the results of the particular study are confirmed by the results of other studies of the same topic.

Obviously few economic evaluations would pass such a stringent test of their methodology [Gerard, 1992, Udvarhelyi et al., 1992]. Rather, the 10 question checklist should be regarded as a

methodological 'gold standard' to which analysts should aspire. In the same way that we do not abandon medicine because it occasionally fails, we should not abandon economic evaluation as an aid to decision making because some of the studies have methodological imperfections.

2.3 Current Methodological Issues in Economic Appraisal

Although there is agreement on many of the general methodological points, there are still many areas where more research is required. These have been outlined by Drummond et al. [1993]. (See Table 2.1.)

First, there is a low level of agreement over whether to include indirect costs and benefits in the evaluation. Some analysts argue that these represent relevant resource changes equivalent to those in indirect costs. Other analysts argue that production may not actually be lost when someone is absent from work owing to illness. In the case of short term absences the work may be covered by others. In the case of long-term absences the worker may be replaced. Certainly there are doubts whether the traditional method of measuring indirect costs and benefits, gross earnings, is appropriate.

Secondly, analysts disagree about whether the health care costs in added years of life should be included in the analysis. Suppose a hypertension screening programme extends individuals' life through a reduction in fatal strokes. If, on the one hand, the life years gained are counted as a benefit of the programme it seems fair to include, on the cost side of the equation, the costs of the health care those individuals will consume (e.g. in treatment of arthritis or cancer in later years of life). On the other hand it might be argued that the decisions to treat the arthritis

Table 2.1: Examples of the Level of Agreement among Economists on Particular Methodological Issues

High level of agreement	Low level of agreement
Terminology of economic evaluation (e.g. cost–effectiveness analysis, cost–benefit analysis, etc.)	Inclusion of indirect costs and benefits
Superiority of marginal costing	Inclusion of health care costs in added years of life
Importance of considering alternatives in an evaluation	Choice of discount rate for health benefits
Importance of analytic viewpoint and the need to consider the societal viewpoint	Method of measuring the utilities of health states
Discounting (in principle)	Incorporation of considerations of equity in economic evaluations
Importance of performing a sensitivity analysis	Inclusion of intersectoral consequences of health care programs

and cancer should be evaluated separately, since they are not an inevitable consequence of the hypertension screening programme.

Thirdly, there is a low level of agreement about the discount rate for health benefits. (Discounting to present values is the approach used to adjust for the fact that costs and benefits occur at different points in time.) The most widely–used convention is to discount both costs and benefits by an annual rate of 5 per cent. However, this has no theoretical foundation [Krahn and Gafni, 1992]. Also, there has recently been a debate about whether health benefits should be discounted at all [Parsonage and Neuberger, 1992; Cairns, 1992]. This debate is of more than just academic interest. The discount rate for health benefits makes a big difference to the economic attractiveness of preventive technologies, since the majority of their benefits are in the future.

Fourthly, there is a low level of agreement about how to value the intangible benefits of health technologies, whether expressed in money terms (through assessment of willingness–to–pay), or in terms of preference values for health states.

In the case of health state preference values (often called utilities), Drummond et al. [1993] suggest that there is still considerable debate concerning:

- the appropriateness of the QALY measure, as compared with the healthy years equivalent;
- the differences between the methods for eliciting values of health states (e.g. rating scale, time trade–off, and standard gamble);

- the likely differences in valuations of health states between different groups (e.g. doctors, patients and the general public) and across countries; and

- the potential for developing generic indices, with prescaled health utility values, that would avert the need to value every health state independently.

Finally, there are other areas of study methodology where more discussion is required. These include the incorporation of equity considerations in economic evaluations and the inclusion of intersectoral consequences of health care programmes. These would be particularly important if large technological changes were being considered. They are probably of lower significance when alternative treatments for a given health care condition are being compared.

2.4 Policy Issues Amenable to Economic Analysis

In seeking to increase the relevance of economic appraisal, it is important to consider the link between health technology assessment, in particular economic appraisal, and health care decision making more generally. Health care systems in different countries vary widely and it is not possible to devise general rules for how this should be done. However, Haan and Rutten [1987] outlined a number of mechanisms, or policy instruments, for encouraging a more rational diffusion and use of health technology. In this section a number of these policy instruments for using economic appraisal results are discussed, with relevant examples from a number of countries. In practice more than one approach is likely to be required, the exact mix depending on the overall organization of health care in a given country.

2.4.1 Planning of specialist facilities or specific technologies

This mechanism is obviously most relevant to the 'big ticket' technologies and to those health care systems where central or local government does have the power to influence decisions about the location of (say) open heart surgery units, neonatal intensive care or specialist diagnostic facilities. Although such power exists primarily in predominantly public health care systems like the British NHS, or those with a national health insurance plan, there may also be opportunities to influence decisions in 'liberal' health care systems if the development of specialist facilities either requires significant medical research funding or a large number of patients whose bills are paid by the government.

There are a number of ways in which economic analysis could contribute to decisions about the number and location of specialist facilities. First, there is the question of optimum size of such facilities, where information about the shape of the long run average cost curve would be useful, although presumably one should not neglect the costs (borne by the health care system or patients) in travelling to specialist facilities. This suggests examination, by economic analysis, of another choice; that of transporting patients to specialist facilities as an alternative to providing more facilities closer to a greater number of centres of population. There has been surprisingly little examination of these issues by economists.

However, the major problem in planning a rational distribution of specialist facilities is that medical technologies continually develop. Therefore, one might find that through time previously unsuccessful procedures improve in effectiveness and that the range of clinical indications for effective medical intervention expands. Thus it is necessary to adopt an iterative approach to the

planning of facilities and the use of economic evaluation. The stance taken by the UK government on heart transplants was that no more units would be funded until the costs and benefits of treatments given in the existing two units had been investigated [Buxton, 1987]. (Such restrictions on the spread of new technology can often be justified on clinical as well as economic grounds. A clinical team needs to perform a minimum number of procedures in order to develop its expertise.)

Finally, whatever one were able to achieve in the field of planning the number and location of specialist facilities, one would still need to influence medical policy within such units. Even though the number and distribution of units might be linked to the likely 'need' in the population as defined by cost–effectiveness criteria, the units might still be filled by 'inappropriate' cases; that is, patients with clinical conditions where the benefits from treatment do not justify the costs when compared to alternative uses for the same resources. This has certainly been the case with high technology diagnostic facilities such as C–T scanners. Therefore attention also has to be paid to the use of economic evidence in developing medical audit and utilization review schemes (see 2.4.6 below).

2.4.2 Excluding technologies from public reimbursement

This mechanism can be applied both to big ticket and small ticket technologies. A number of countries have organizations which decide on the suitability of new technologies for public funding. In addition, health care insurers in some countries are guided by a central organization (e.g. the Sickness Fund Council in The Netherlands). In principle such agencies could consider evidence on costs alongside effectiveness when taking decisions about the size of the health insurance 'envelope'.

There is some evidence that this is beginning to happen, especially in The Netherlands where the Health Insurance Executive Board has commissioned a number of economic evaluations [Haan and Rutten, 1989]. However, the problems should not be understated. It is important that such bodies have clear remits with respect to the consideration of cost–effectiveness. Also, whether or not a particular technology is the most cost–effective approach to the treatment of a patient may often depend on the specific circ mstances, such as the severity of the patient's condition or the diagnostic and therapeutic procedures that have already been applied. For example, is it cost–effective to undertak a magnetic resonance scan when a C–T scan has already been performed? It is difficult to envisage how regulatory bodies could do more than make general judgements about the costs and benefits of health technologies. However, they might engage in more analysis of a 'what if?' type. That is, would the new technology yield benefits in excess of costs even if one assumed that there was likely to be some inappropriate use? There have been some retrospective analyses of the net economic impact of certain health technologies, such as the drug cimetidine [Bulthuis, 1984]. Perhaps there should be some prospective analyses, with a commitment to monitor the situation as the new technology diffuses.

The other major difficulty facing regulatory agencies is that economic data on new technologies are often lacking. This means that a specific study needs to be commissioned at a time when the agency may be under pressure, from health care professionals, the public or the manufacturer of the technology, to make a decision. To some extent this problem could be ameliorated by assembling economic evidence earlier in the development of technologies, perhaps by undertaking economic appraisal alongside clinical trials [Drummond and Stoddart, 1984].

Recently, in Australia and Ontario (Canada) draft guidelines have been proposed for the pharmaceutical industry on the preparation of economic analysis to be included in submissions to the government committee deciding on the reimbursement of pharmaceuticals [Drummond, 1992a]. A new drug will have to show that it gives good value for money before being listed on the national or provincial formulary. These policy initiatives are in their early phases and it is too early to predict the final outcome. However, they demonstrate that governments are beginning to take value for money evidence seriously and that guidelines for undertaking studies can be specified.

The debate has recently been broadened in Canada, whereby thresholds for the adoption of health technologies have been proposed, defined in terms of cost-effectiveness. For example, a new technology costing more than an extra $100,000 per quality-adjusted life-year gained is considered to have only a weak case for adoption [Laupacis et al., 1992]. Quite apart from the existence of formal requirements many pharmaceutical companies are themselves assembling evidence in support of their products and this has been encouraged by the government in the United Kingdom [Drummond, 1992b].

2.4.3 Reforming payment schemes for health care institutions (especially hospitals)

One of the most significant reforms over the past few years has been the movement towards prospective reimbursement for hospitals, the most well-known scheme being that based on diagnostic related groups (DRGs) operated by Medicare in the USA. Romeo et al [1984] have examined the impact of three prospective reimbursement schemes on the diffusion of five 'little ticket' technologies, all of which had an acquisition cost of less than $100,000. (These were

electronic fetal monitoring, volumetric infusion pumps, upper gastrointestinal fibreoptic endoscopes, automated bacterial susceptibility testing and centralized energy management systems.) Their results were largely encouraging; they noted that in New York State (the most restrictive of the three schemes examined) there was more effect on the extent of adoption of technology rather than on the initial decision to adopt (as measured by the availability or delay variables in the model). Both Romeo et al. and the technical memorandum produced by the Office of Technology Assessment [1983] point out that the long run viability of any DRG–type payment system depends on its ability both to adapt to, and encourage, appropriate technological change in medicine. Therefore, the calculation of reimbursement rates should take note of evidence on the relative cost–effectiveness of alternative treatment methods for clinical conditions and this evidence should be more actively disseminated. At present there is perhaps too much of a tendency to set the rates and leave the hospitals to cope with the consequences. This is potentially inefficient, especially if hospitals take decisions based on their own costs and benefits, rather than those of the community at large. This reaffirms the importance of performing economic evaluations from a number of viewpoints, including the societal viewpoint, so that appropriate incentive structures can be devised for the key actors in the health care system, as was mentioned above.

2.4.4 Encouraging budgetary reform within institutions

An interesting development here has been the experimentation with clinical budgeting in the UK and elsewhere. Here a clinical department or 'firm' is subjected to budgetary control, and given incentives to search for more cost–effective procedures by being allowed to redeploy a proportion of the savings made. Although the results from such experiments have been mixed, the evidence is currently promising enough for them to proceed.

The role for economic evaluation in such schemes would be in the discussion of the clinical plan and budget for the coming year. Here it would be possible to discuss the evidence on (say) the cost–effectiveness of day–case surgery and to consider the implications of its adoption. To a lesser extent clinicians may also be stimulated to undertake their own economic appraisals of new clinical procedures.

2.4.5 Changing payment systems for health care professionals

In countries where physicians are paid by fee–for–service, or where special additional payments are made for some services, there have been concerns that the payment system leads to inappropriate use of technology. Some analysts suggest that this system leads to supplier–induced demand [Evans, 1974]. Others are concerned that the rewards to the physician may be relatively higher for time spent using expensive technology than for time spent talking to the patient or counselling. Given these concerns, it is surprising that there has been relatively little study of fee schedules and few attempts to change them. For example, it would be interesting to study whether there are consistent incentives (implicit in the schedule) to encourage physicians to spend their time using expensive technology, whether physicians are consciously aware of these incentives and whether they influence their behaviour. This would be an important precursor to studies of how the fee schedule could be used more aggressively to change clinical practice in the direction of greater cost–effectiveness, by withdrawing payment for procedures known to be inefficacious and by offering attractive fees for procedures for which benefits are known to exceed costs. The latter approach can also be useful in health care systems where the predominant method of payment of physicians is by salary.

2.4.6 Developing medical audit and utilization review schemes

A few years ago the World Health Organization (Regional Office for Europe) reviewed the schemes operating in a number of countries, with a view to the potential for incorporating economic criteria [WHO, 1981]. Two schemes were of particular interest: Scandinavian Model Health Care Programmes, where guidelines are developed for the management of key diseases such as hypertension; and the medical audit schemes developed by the National Association for Quality Assurance in Hospitals in The Netherlands (the CBO), where groups of physicians are provided technical support to review local clinical practices. In both cases there was evidence that economic criteria could be incorporated in the development of guidelines and that attempts were being made to assess the impact of guidelines in terms of cost–effectiveness.

There are other examples of economic appraisal being used to help develop guidelines recommended by medical bodies, such as the work by Eddy [1980] on cancer screening and that by the Royal College of Radiologists [1980] in the U.K. on routine skull X–rays for patients admitted to the Emergency Room with head injury. Against the background of increasing pressure on health care budgets, there is no reason why more studies could not be encouraged. The influence of professional bodies and medical opinion leaders has probably been under–exploited by those undertaking economic evaluation and those funding health services research. In this connection those interested in a more rational diffusion and use of health technology could learn much from pharmaceutical companies and medical equipment manufacturers who target opinion leaders with their promotional activities.

2.4.7 Introducing co-payment for service users

Health care systems differ in the extent of co-payment (charges) for service users. One approach to co-payment would be to reimburse technologies only to the level at which the government considered them to be cost-effective and then to call upon the service user to contribute any excess. This is similar to the approach being followed in The Netherlands and Germany in setting 'reference prices' for pharmaceuticals. Here the government sets a reimbursement level for a therapeutic class of drugs (e.g. beta blockers) or a particular clinical indication (e.g. treatment of acute migraine attacks). If some drugs are more expensive than the reference price, the patient would have the choice of paying the difference, or using another drug priced at the reference price. To date most reference price systems apply to drug classes where generic products are available.

Whilst potentially attractive to governments facing budgetary restrictions, the use of co-payments needs to be considered very carefully [Barer et al., 1982]. In particular, it is important to assess whether co-payments are likely to be regressive (i.e. penalizing the poor). If the existence of charges deters the poor from seeking appropriate care this may be more inefficient in the long run. However, where there is no difference in effectiveness between high cost and low cost alternatives and where price regulation for a given technology is difficult, co-payments may have a place.

2.4.8 Encouraging competitive arrangements in the health care system

Some European countries, most notably the United Kingdom and Sweden, have considered ways of encouraging competition within their publicly–funded health services. (Competition has long been debated within private, market–based systems such as that existing in the USA.)

In the reformed British National Health Service a separation has been made between the purchasers of services (e.g. local health authorities and 'fund–holding' family physicians) and the providers (e.g. hospitals). The idea is that, in an 'internal market', services will be purchased and provided according to contracts. The reforms give considerable scope for the use of economic evaluation. For example, purchasers could use cost–effectiveness data to decide whether or not to place a contract for a given service or technology, and to decide upon the appropriate method of treatment to be specified in the contract. Also, in a competitive environment providers have an interest in knowing which treatment technologies are more cost–effective, since adoption of these give the best chance of winning more contracts [Henshall and Drummond, 1992].

As with some of the other recent policy initiatives, it is a little too early to give an assessment of the British reforms, or whether they do, in practice, lead to more use of technology assessment. However, the government, in its new research and development strategy, has put an emphasis on the dissemination of research evidence and has commissioned the production of a series of 'effectiveness bulletins', which give guidance to purchasers on the likely cost–effectiveness of services such as screening for osteoporosis, stroke rehabilitation, infertility treatments, surgery of the middle ear in children, treatment of depression, cholesterol lowering and breast cancer treatment.

This review demonstrates that evidence on the cost–effectiveness of health technologies can be profitably used. The main remaining challenges are to produce economic data in a timely fashion and to make a better link between economic evaluation and health care decision making.

Progress towards meeting these challenges is more likely to take place if health technology assessment is seen within the broader context of health policy. In particular, study results are more likely to be obtained in a timely and relevant fashion if medical and health services research policy is reoriented towards this goal, by insisting on inclusion of the appropriate economic analysis alongside clinical trials. Also, health care decision making, and the incentives facing key decision makers should encourage, rather than discourage, the use of economic data about health technologies.

2.5 Increasing the Relevance of Economic Appraisal Results

2.5.1 Maintaining methodological standards

The methods of economic appraisal were discussed earlier. From a policy perspective it is important to note that a number of accepted methodological principles have emerged. These have been summarized by Drummond et al. [1987] in the form of a 10–point checklist. This can be used by decision makers wishing to assess the quality of published studies. More recently there has been discussion of standardization of economic appraisal methods at the European level [Drummond et al, 1993]. If this effort is successful it will greatly assist policy makers wishing to interpret the results of studies undertaken in different settings. There have now been many economic appraisals of health technologies, particularly in Europe and North America.

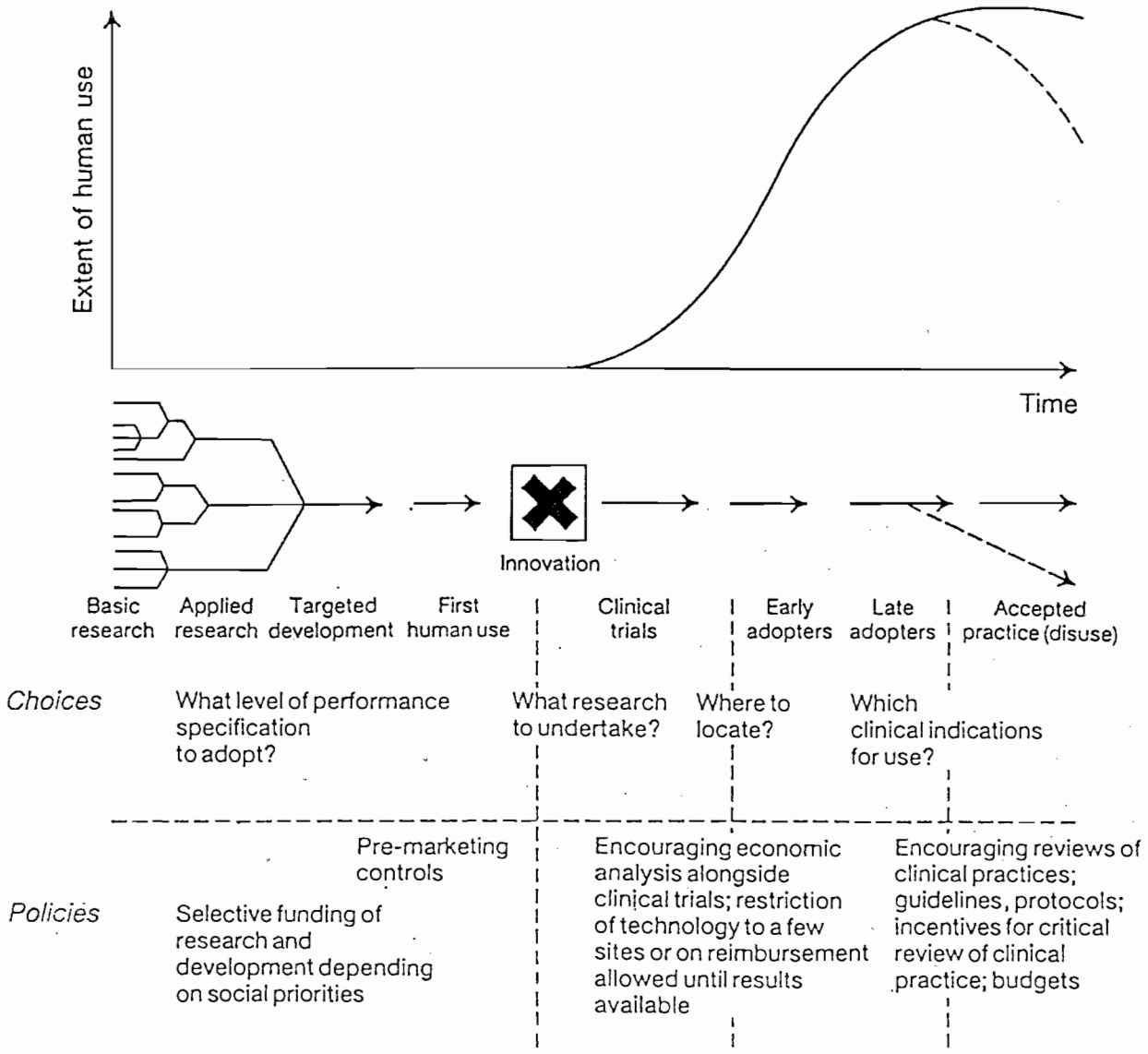
Those undertaking assessments of health technology usually pay particular attention to the methodological quality of studies. However, it is possible to overstate the importance of good study methodology, since this is usually only a necessary, but not sufficient, condition for the use of a given study. It is unlikely that good methods alone will convince the opponents of the recommendations of a particular assessment. Rather, good methods are more important in defending the study from attack by those who oppose the conclusions. Where the conclusions of an assessment are generally popular, confirming many individuals' prior beliefs about a particular technology, methods are less likely to be subjected to close scrutiny.

2.5.2 Producing economic evidence in a timely fashion

In considering policies for health technology assessment it is important to ensure that the appropriate evidence is available at the relevant points in a technology's lifecycle. A typical diffusion curve for a health technology is shown in Figure 2.2 [Banta et al., 1981]. It is important that economic evidence is assembled before wide adoption of a technology, although the technology may be previously used on an experimental basis.

A major vehicle for assessing the efficacy and effectiveness of health technologies is the controlled clinical trial. Therefore attention has centred on the need to undertake economic analysis alongside clinical trials and on the ways of minimizing the amount of unnecessary effort [Drummond and Stoddart, 1984]. There are a number of methodological difficulties, arising because of the atypical nature of the setting for many trials (e.g. specialist centres), the clinical alternatives evaluated (e.g. placebo or baseline therapy rather than current best practice), the short

Figure 2.2: The Diffusion Curve for Health Technology: Choices and Policies



(Adapted from Banta et al., 1981.)

period of follow-up and the small sample size, which may be adequate for assessment of the primary clinical endpoint but not for some of the key economic variables [Drummond and Davies, 1991].

However, these problems are gradually being overcome and some countries [Ontario Ministry of Health, 1982] have formed procedures whereby clinical research submissions are screened in order to assess whether an economic evaluation would be appropriate. Also there are now a number of examples of Ministries of Health providing funds to ensure that adequate economic evaluations are undertaken of important new technologies, such as continuous hyperfractionated accelerated radiotherapy [Morris and Goddard, 1993].

It is often argued that, for an assessment to have an impact, it needs to be timely. The timing of health technology assessments is no easy matter. Buxton [1987] has argued that, because of the rapid pace of technological change in medicine, it is 'always too early, until suddenly it's too late'. Certainly, there are key stages in the diffusion of technologies where important decisions are to be made and where study results are more likely to be used (see Figure 2.2). These include decisions about allowing entry of the technology into the health care system, the placement and distribution of specialist units and the granting of public reimbursement. In such situations it may be better to provide timely, if imperfect, data on costs and benefits, rather than definitive data after the decision has been made. However, it remains important that the imperfect data are not seriously misleading in their implications.

The other aspect of timeliness relates to the broader economic and political environment within which technology assessments are conducted. For example, it is easier for governments to make controversial decisions at some stages of a parliament rather than at others. Similarly, the data from a technology assessment may become available at a time when other recent events suggest a particular decision. It is clearly wrong to view the results of health technology assessments and their implementation as being totally independent of the decision making context prevailing at the time they become available.

2.5.3 Increasing the local validity of study results

There is a general shortage of resources for health technology assessment and it will not be possible to undertake a given study in every setting. For example, with emerging technologies, decision makers are often reliant on assessments undertaken in the United States. In such cases the opponents of study conclusions may argue that the situation prevailing locally is different from that in which the study was undertaken. There are often differences in clinical practice, local health service organisation or relative prices that could affect whether a given technology is cost–effective in a given setting. The obvious solution would be to repeat the study in full, using local data; but where this is not feasible it may be possible to extrapolate from results obtained elsewhere – where necessary taking account of major differences between setting by making minor modifications in data or methods. For example, one study of a new drug technology has been undertaken in a way that would facilitate such extrapolation [Drummond et al 1992]. (This study is discussed in the case study on pharmaceuticals in Chapter 5.)

2.5.4 Increasing the decision maker involvement in the study

In a review of health technology assessments undertaken in the United Kingdom, Drummond and Hutton [1987] noted that the vast majority were conducted by independent researchers with no obvious link to the decision making process. Whereas the independent researchers may minimise the potential for bias in study methods, it is much more likely that the results could be ignored by key decision makers, either because they are unaware of the studies concerned, or because they do not address what the latter define as the relevant issues. If the decision maker is involved in the study, perhaps by commissioning it or being involved through an advisory committee mechanism, there may be a greater chance of impact. If this process works well, the study will be more likely to address the relevant questions. Furthermore, having been involved in the design and conduct of the study, it may be harder for the decision maker to distance himself or herself from the conclusions, or fail to act on them.

Of course, decision maker involvement is no guarantee that the study will have an impact. Ways can be found to ignore 'inconvenient' results. Indeed, decision makers may want to apply criteria that were not addressed in the health technology assessment, such as the impact on employment in a depressed region if the plant manufacturing a particular technology were to close.

2.5.5 Improving the dissemination of study results

If technology assessments are to have an impact then the results of studies need to be widely known. The results of studies undertaken by independent researchers are not widely disseminated; often the researchers view publication in a learned journal as their main aim. Other

dissemination activities, such as interviews with the media or seminars for key decision makers, generally have lower priority than beginning the next study. Decision maker involvement can often encourage dissemination, since certain activities can be specified as part of the research contract. However, there are also concerns that certain sponsors might suppress results if the research contract allows this. For example, this issue has recently been raised in the context of research sponsored by the pharmaceutical industry [Hillman et al 1991].

2.5.6 Taking note of the availability of policy instruments

In order for studies to have an impact, decision makers need to have the appropriate mechanisms for influencing the diffusion and use of health technologies. Haan and Rutten [1987] have specified a range of policy mechanisms within the European Community, Hailey et al. [1990] have outlined some possibilities for Australia. It was pointed out above that there is no shortage of available instruments, but it is important that researchers consider how the results of their study could be used.

2.5.7 Recognising the conflicts and incentives surrounding the study

It has to be recognised that there are many actors in the health care system, often having different objectives. For example, if the sponsor of a particular technology assessment is the government or third party payer, it is unlikely that the sponsor's interests will be the same as those of the technology's manufacturer, or the physicians who may use the technology, those whose services may be replaced by it. In the (rare) cases where there is a commonality of interests, an evaluation producing results supportive of those interests is highly likely to have an impact.

Conversely, if the evaluation produces results counter to the interests, it is much less likely to bring about change.

In the more usual cases, where there is a plurality of interests, two factors are likely to encourage an impact. First, it is important that the assessment identifies the costs and benefits according to the key perspectives. For example, if physicians are likely to lose income as a result of the introduction of a new technology, this is important to know, so that appropriate action can be taken. Secondly, it is thus important that attention is paid to the incentives facing the key actors. If the total benefits from a given technology outweigh the total costs (when judged from a societal perspective), can the incentives be arranged so that no–one is worse off as a result of its introduction? Although an obvious point, the attention paid to key actors' perspectives is usually slight in health technology assessments. The interests of the key actors should be considered and policies developed so that what is beneficial from a societal perspective is also in the interest of each major group.

2.6 Conclusions

There is considerable potential for the use of economic appraisal in developing policies for the rational diffusion and use of health technology. The recent reforms in many European health care systems increase this potential. However, it is important that policies for encouraging the use of economic appraisal are not considered in isolation from health care policy more generally. In particular, it is important to consider the range of policy instruments for encouraging efficient use of health technology and the contribution that economic evidence can make. Also, it is important to consider the ways in which economic evidence can be made more relevant to policy making.

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3. INTRODUCTION TO THE CASE STUDIES

Policies to guide the diffusion of health care technologies are quite different across health care systems and involve quite different actors. Hurst [1991] suggests that health care systems can be described as being made up from seven different mixtures of major subsystems of finance and delivery of health care. One distinction is related to the finance side and consists of three categories: out–of–pocket payment for health care, voluntary or private health insurance, usually with choice of insurer, and compulsory or public health insurance usually without effective choice of insurer. The latter two categories may be combined with one of three methods of paying providers: indirect payment of providers through reimbursement of patients, direct payment of providers by contract and payment of providers by global budget and salaries in a vertically integrated system. These characteristics in finance and delivery determine the degree and type of government regulation in these health care systems, either centralized or decentralized, and, as often in public insurance systems, with a delegation of power to quasi–governmental institutions.

Depending on the character of the system and the actors involved in health policy, the role of economic evaluation will be different. Its role will be clearer when firm regulatory mechanisms are in place than in the case of a decentralized system, where contracts between local financers and local providers determine the degree of diffusion of health technologies. The specifics of resource allocation in a particular health care system may even determine the type of economic analysis needed. The perspective from which a study is undertaken differs according to whether central government or a local health care insurer is the principal user of its results. In the former case a societal perspective (taking all costs and consequences for all parties in society into account) will

be required, while in the latter case the insurer will mainly be interested in the costs to be reimbursed from his own budget or the savings to his benefit. Furthermore, in a competitive environment there is a tendency to rely on cost-benefit analysis (willingness-to-pay determining the value of outcome), while in a socialized system the objective is to maximize health outcome given a public budget and than cost-effectiveness or cost-utility analysis is more appropriate.

Health care systems will also differ with respect to their equity- and efficiency characteristics [OECD, 1992; van Doorslaer et al., 1993]. In systems with predominantly private finance there may be problems with respect to the availability of health care services and with distributional objectives, which may be redressed by subsidizing specific health care programmes, and encouraging the use of guidelines and protocols to guarantee cost-effective and equitable use of such programmes. In national health systems at the other end of the spectrum, where there is direct payment of providers by a global budget and salaries in a vertically integrated system, there may be problems in achieving micro-economic objectives. Although in this case emphasis will be on regulation by directive, reforming payment schemes for health care institutions and introducing budgetary mechanisms within these institutions may help to improve micro-economic efficiency. Again, different studies will have to be performed in support of these different policy instruments, the selection of cost-effective health care programmes requiring other information than the development of clinical budgets within a health care institution.

We should also be aware that different health care systems provide different opportunities for economic appraisal when considering the availability and degree of detail of information from existing data registries and accounting systems within institutions. In the case of indirect payment of providers through reimbursement of patients and direct payment of providers by contract, there

is much more information on specific health care activities available than in a system where there is direct payment of providers by global budget and salaries. In cost-effectiveness analysis comparing two or more specific strategies it is necessary to collect specific data in the context of the study itself, but in assessing the efficiency of more global programmes it can be profitable to be able to use data from existing registries. Such data may also be useful for generalizing from the results of detailed studies.

In Chapters 4 – 6 three case studies will be presented. These were chosen in order to provide an insight into the role of economic appraisal for various types of health policy decisions and to give the opportunity to consider different methodological issues. The first case study deals with the decision to initiate a national screening programme, in this case a programme of screening for breast cancer. In this case we will consider a Dutch economic evaluation study on breast cancer screening and see how the results of this study were used to decide on the initiation of a national screening programme in the Netherlands. The interesting feature of this case is that the study proved also to be influential in monitoring the gradual development of the programme in practice. The second case is concerned with decisions whether to reimburse a pharmaceutical product from the public budget. This will be illustrated by considering economic appraisals of the use of prostaglandin E_2 to induce labour and of the prophylactic use of misoprostol in arthritis patients taking non-steroidal anti-inflammatory drugs who experience abdominal pain. Finally, the last case concerns the decision to develop heart transplantation programmes both in the U.K. and the Netherlands, where economic evaluation studies were initiated by the UK government (DHSS) and the Health Insurance Executive Board respectively to support a policy decision on the initiation of the programme, the size of the programme and the number of heart transplant centres involved.

Table 3.1 shows a number of characteristic features of the case studies selected. As will be demonstrated in the next sections, the case studies provide ample opportunity to discuss the link between policy and research, the role of the funding body, some extensions of the economic evaluation framework and a number of relevant methodological problems.

Table 3.1: Selection of Case Studies

	Case I **breast cancer screening**	**Case II** **pharmaceuticals**	**Case III** **heart transplant**
type of policy decision	initiating preventive programme	reimbursement of pharmaceuticals	planning of specialist facility
funding of study	government or public insurance agency	pharmaceutical industry	government or public insurance agency
particular issues explored	organizational aspects of a national programme	comparisons of competitor products	economies of scale and impact on future public spending
methodological features	simulation of alternative strategies according to frequency of screening and age groups	extrapolation of clinical trials results using a decision-tree	construction of a reference strategy and development of a scenario-model

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4. INITIATING NATIONAL SCREENING PROGRAMMES: THE CASE OF BREAST CANCER SCREENING

4.1 Introduction

The decision to initiate a national screening programme, which is generally taken at the level of central government, has major implications. Usually, the initiation of such a programme has large financial consequences, which have to be met entirely, or at least for a major part, out of the public budget. Furthermore, there is an important ethical issue related to the fact that people who are generally in good health are urged to use health care services (at least diagnostic services), that may not be of much use for the majority of individuals. Some policy makers may consider this as an additional obligation to consider carefully the costs and benefits of such a decision. In addition, the economic appraisal of a screening programme is complex as it requires the assessment of a chain of activities concerning the organization of screening, diagnosis, further assessment and consequent treatment. A lot of information is necessary to assess the costs and benefits of each phase and clearly in the final analysis these costs and benefits should be considered simultaneously. Furthermore, the effects of screening and/or consequent treatment may not become apparent until some time in the future, thus introducing additional uncertainties concerning technological advances in diagnosis and treatment.

Given the weight of such policy decisions it is not surprising that economic evaluation has been relatively prominent in those cases where decisions about major screening programmes have been taken. In a recent review on the status of economic appraisal of health technology Davies et

al. [1993] observed, that 54% of the studies reported to the EC network on the methodology of economic appraisal of health technology were studies on prevention, which were generally funded by government or social insurance organizations. Some countries have formulated explicit policies regarding the use of economic analysis in deciding about major health care programmes. An example of this is the policy of the Health Insurance Executive Board in the Netherlands, which developed a three step procedure to decide about major health care programmes in the mid eighties: (i) diffusion of the new technology should be limited and controlled; (ii) an economic appraisal should be carried out simultaneously; and (iii) a decision about reimbursement of such new technology or programme should be made taking into account the results of the economic appraisal.

The decision in 1990 to initiate nationwide breast cancer screening in the Netherlands was made according to this procedure. In the mid eighties two pilot projects in Utrecht and Nijmegen began and the Erasmus University Rotterdam was asked to take part in the evaluation of the results of these pilot projects and to report about the costs and effects of nationwide screening. The final report on the cost-effectiveness of the screening programme was issued in April 1990 and formed part of the evidence on which the health authorities decided to initiate nationwide screening in the Netherlands [van der Maas et al., 1989; van Oortmarssen et al., 1990; de Koning et al., 1991 and de Koning, 1993].

We have selected this study to illustrate the role of economic appraisal in decisions about preventive programmes for three reasons. First, the study illustrates the difficulty of assessing a complex programme consisting of a chain of activities. Secondly, in this case specific attention is given to the organizational problems associated with introducing such a large programme and to

the fact that the choice of organizational arrangements affects the cost–effectiveness of the programme. The third reason, related to the second, is that the study has not only proved to be useful for supporting the decision to initiate the screening programme, but the study is continuously updated and used as a tool for monitoring the implementation of the programme.

In the next section we will provide some background information about the study and give some global results emphasizing the way that these have been used in the decision process. Then we will highlight some of the organizational aspects of introducing such programme. Finally we will see how the study is used to monitor and guide the implementation phase of the programme.

4.2 Cost–effectiveness of Breast Cancer Screening

As Drummond et al. [1986] commented earlier, in most cases evidence on the effectiveness of prevention programmes is either obtained from "before and after" studies, or by extrapolating from evidence obtained under more ideal circumstances such as randomized trials. This study is no exception in that it uses several sources of information to come to a conclusion about cost–effectiveness of the programme. The impact on mass screening on incidence, stage of disease distribution and survival was estimated from the results of trials in New York, Sweden, Nijmegen and Utrecht, and was reanalysed using detailed data from the Dutch pilot experiment. These experiments were also an important source of the data for the cost calculation: extrapolation of cost data from the Dutch trials provided the empirical basis for the estimation of the required manpower and facilities for a national programme. Data from national registries were used to estimate the costs of assessment of suspected breast cancer cases and the costs of consequent treatment.

An important feature of the study was the use of a computer simulation package MISCAN [Habbema et al., 1987]. This package was developed for analysing and reproducing the observed results of screening projects and for predicting the future effects and costs of alternative modes of screening. It is based on a three stage division of the development of invasive breast cancer, the stages reflecting the size of the tumour. This simulation programme allows the calculation of a cost-effectiveness ratio for target groups, which differ with respect to age group, and for different intervals between inviting the women to be screened.

De Koning et al. [1991] report on the cost-effectiveness ratio of introducing breast cancer screening in the Netherlands. For the calculation of the costs it is assumed that the screening programme will have started in 1990 and that it will end in 2017. The costs and effects of mass screening in this period, occurring after 2017, are computed until all women who may have benefited from the screening programme will have died. Table 4.1 provides the results of the study for different screening policies, using different age groups and different screening intervals. This allows comparison, for instance, with the U.K. policy of three yearly screening of women age 50 - 65. The two yearly screening programme for women aged 50 - 70 is predicted to detect 26% of all diagnosed breast cancers in the population. The total costs for screening are US$ 300 million and the additional costs of treating and following up more women earlier are US$ 72 million. On the other hand the expected decrease in the costs of treatment is US$ 128 million. The table shows that the screening option for age group 50 - 65 with an invitation interval of three years is most cost-effective, followed by the option for age group 50 - 70 and a two year interval. As the number of QALYs gained in the latter case is much higher than in the former, and the cost-effectiveness ratios for both options are rather favourable, the initial preference by field experts for the age group 50 - 70/2 year option could be defended on the basis of these results and therefore

this scheme was selected as the basis for the Dutch screening programme.

Table 4.1: Effects on Mortality, Costs, Cost-effectiveness and Cost-utility for Different Breast Cancer Screening Policies (1990 - 2017) in the Netherlands.

5% discount rate and costs in millions US$ (unless stated). Cost amounts are expected differences between situation with and without screening (1990 prices).

Age group screening interval	50 - 70 2 yr	40 - 70 2 yr	50 - 70 1.3 yr	50 - 75 2 yr	50 - 65 3 yr
Breast cancer deaths prevented[1]	17,000	17,800	19,800	19,450	10,800
Life-years gained[1]	260,000	290,000	310,000	275,000	180,000
Cost of screening	300	457	405	310	185
Cost of assessment/biopsy	-10	-62	-12	2	-12
Cost of primary treatment	50	57[2]	55	71	26
Cost of follow-up	22	25	25	27	14
Cost of advanced disease	-128	-131	-145	-145	-80
Difference in costs	233	346	328	265	133
Breast cancer deaths prevented	6,000	6,115	6,780	6,790	3,770
Life-years gained	61,000	64,000	70,000	64,500	41,000
Quality-adjusted life years gained (QALYs)	57,500	59,500[2]	66,000	59,500	39,300
Cost (US$) per life-year gained (CE ratio)	3,825	5,385	4,670	4,100	3,235
Cost (US$) per QALY	4,050	5,815[2]	5,000	4,450	3,400

[1] Not discounted

[2] No age-specific data for treating women < 50

Source: de Koning et al. [1991]

Table 4.2 from de Koning et al. [1991] shows some of the results of a sensitivity analysis, which is necessary in complex studies surrounded with many uncertainties. It shows the effect of some alterations in the baseline assumptions made in the study, because of uncertainty about the costs of treatment of advanced breast cancer, the capacity of the screening units, the characteristics of the mammographic screening test, the frequency of follow–up examinations of treated women, the situation without screening and whether or not to include the cost to the patients and the medical cost for other diseases in life years gained. It is interesting to see that the cost–effectiveness ratio is rather sensitive to the last factor, which is understandable as the target population consists of elderly women who will incur other expensive diseases in the not too distant future. The cost–effectiveness ratio including medical costs for other diseases in life years gained is the relevant indicator for policy makers wanting to confront the benefits of the programme with all future health care expenditure. Note however, that there may be problems of comparison since most studies producing cost–effectiveness ratios do not include this cost item. (See the discussion of this point in Section 2.3.)

Table 4.2: Alternative Assumptions, other than Mortality Reduction, that Influence the Cost-effectiveness of 2-yearly Mammographic Screening of Women Aged 50-70 (prices 1990).

(CE in US$ per life-year gained)

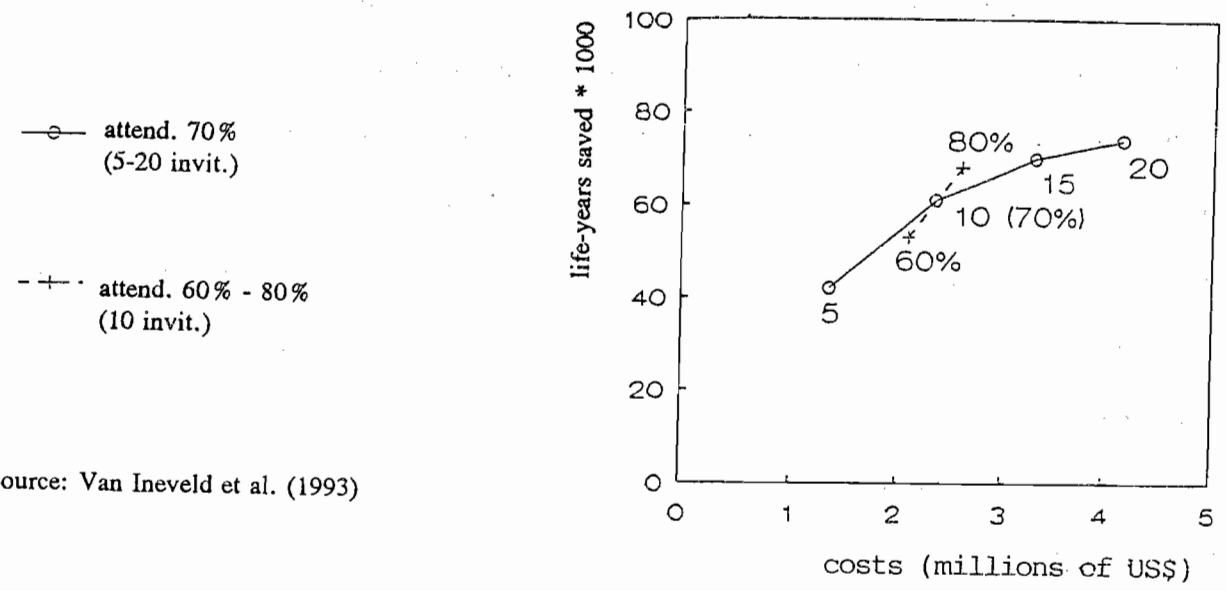
	Actual data or assumption in principal variant	Alternative assumption	CE ratio and % difference with principal variant
Cost of treatment of advanced breast cancer	US $21,000 per woman	25% higher costs	3,300 (-14%)
Capacity of screening units	12,000 women per year	10,000 per year per unit	4,100 (+7%)
Positive predictive value mammographic screening test	51% on average over all rounds	43%	4,130 (+8%)
Follow-up examinations of treated women	Every 3 months in first 2 years	Twice as frequent	4,190 (+9%)
Total costs screening	US $40 per screen	US $43	4,225 (+10)
Non-medical direct costs	Not included	Include travel, time and out-of-pocket costs to women	4,460 (+17%)
Demand for mammograms outside screening programme	Decrease in assessment proportional to decrease in clinical cancers	Only decrease in assessment for preventive reasons	4,465 (+18%)
Indirect costs	Not included	Include medical costs for other diseases in life-years gained	7,250 (+90%)

Source: de Koning et al. [1991]

This short overview of the results of the economic appraisal illustrates the usefulness of this approach for decision-making. The policy maker is able to predict the financial consequences

of the initiation of a screening programme according to the different options; he or she can assess the health benefits and is presented with cost-effectiveness ratios suggesting the relative efficiency of different options from which the choice can be made. These global results are based upon a number of decisions concerning various details of the screening programme, and in the next section we will discuss some of these in relation to the organization of the programme.

Figure 4.1: The Cost-Effectiveness of Breast Cancer Screening with 5, 10, 15 and 20 Invitations per Woman (Aged 50-70 Years) and with 70% Attendance, and the Cost-Effectiveness of a Strategy with 10 Invitations and the Attendance Rate Varying Between 60-80% (discount rate 5%).



Source: Van Ineveld et al. (1993)

4.3 Organizational Aspects

In economic appraisal relatively little attention is given to the way of organizing an intervention or a programme and to the practical problems associated with the introduction of a large new programme [Drummond et al., 1986]. In this economic appraisal specific attention was

given to these aspects, as they were shown to be quite important in the case of breast cancer screening. We will consider two issues here: the organization of the screening unit and the restrictions on the speed of programme implementation.

One of the primary determinants of cost–effectiveness of breast cancer screening is the degree of participation of the women invited. When screening units are fixed sites, travel distances will be large in scarcely populated areas. An alternative to fixed screening units is to make these mobile, and a specific pilot study was performed to assess the performance of a mobile unit. Figure 4.1 shows the relationship between costs and life years saved for different aspects of unit performance [van Ineveld et al., 1993]. The figure suggests that increasing the frequency of invitations is less cost–effective than increasing the attendance rate at a fixed level of 10 invitations in the 50 – 70 age group. Indeed, in the study it could be shown that an increase of the additional costs associated with increasing the attendance rate up to 3% per percent increase in attendance rate could be allowed before the alternative policy of increasing the number of indications becomes more cost–effective. Therefore the additional costs of mobile screening should not be more than US$ 0.75 million yearly percent increase in attendance rate. From the experiment with the mobile screening it was found that mobile screening could raise the participation rate by 6% on average. Comparing these figures with the additional costs of mobile units led to the conclusion that a mixed policy of using fixed units in some areas and mobile units in others was probably most cost–effective.

Another issue dealt with explicitly in this study was the consideration of restrictions on the speed of implementation of the programme. One of these factors is the hiring and training of medical personnel, especially those with diagnostic radiology expertise. By way of simulation it

can be shown that, especially in the second half of the implementation phase of the programme, there will be a shortage of personnel both for the diagnostic and therapeutic radiology activities. Another issue relates to the capacity of facilities offering radiotherapy treatment, using megavolt equipment. The contribution of the breast cancer screening programme to the total demand for radiotherapy treatment was estimated at 2.5% and could only be accommodated by increasing the number of facilities or extending the use time of the equipment. Similarly, the supply of brachy-therapeutic services also needed to be increased, initially by 24% and, in the long run, by 9%. An assessment of the possibility of meeting these requirements provided important information about the maximum speed of implementation of the breast cancer programme.

4.4 Monitoring of the Implementation of the Programme

The study on the cost–effectiveness of breast cancer screening has also been used as a reference point for the evaluation of the first year (1990) of implementation of the programme. This evaluation took place in the beginning of 1992 and produced a number of findings which are helpful in deciding whether expectations about several process parameters of the programme have been met and whether or not it is necessary to institute important changes in the organization of the programme.

van Ineveld [1992] first considered the effects of the screening and compared observed values with those found earlier. The observed participation rate was 72% (70% in the initial study), 1.4% of the women were referred for further assessment (1.6% in the initial study) and the predictive values for referral and operation were 42% and 65% (41% and 54% in the initial study). These and other figures led to the conclusion, that there are no reasons to make

fundamental changes in the important characteristics of the programme.

The approved budgets for the programme in the years 1990 – 1992, however, were considerably higher, amounting to a difference of 26% in 1992 and a projected difference of 49% at full implementation. The detailed analysis of the differences between observed and projected costs produced the following findings:

- in some regions women older than 70 were also screened (at least 11 screens instead of 10), which led to a 12% increase in the volume of screens;

- several decisions to increase the quality of the programme were taken (e.g. a more advanced semi mobile screening unit, more costly training programmes and daylight film processing equipment). These decisions increased expenses considerably and were not sufficiently supported by cost–effectiveness considerations;

- in a number of cases (e.g. equipment, medical activities) budgets were exceeded in ways which could not be accounted for;

- in sum, the difference of 49% was made up of 12% increased volume, 32% increased prices (12% general price increase and 20% specific) and a 5% combined price and volume increase.

The conclusion was that the results so far were satisfactory, but focus had been on effectiveness and speed of implementation rather than on the containment of the costs. A number

of suggestions were made to economize on the use of resources in the programme and to change policies for the execution of the programme, leading to savings in the long term (1997) of five million US dollars.

4.5 Conclusion

We have seen that a detailed study of the costs and effects of a complex screening programme is quite difficult and also rather resource intensive. The return on investment from such a study does not only have to come from supporting the initial decision to implement the programme, but also from determining the way in which it should be implemented and from monitoring the programme during implementation. But still, health authorities in a particular country may ask themselves whether a study in their country should be performed when there is information available about the relative efficiency of such a programme from other countries. This question was addressed by van Ineveld et al. [1993] when they used this study to explore the differences in cost-effectiveness of breast cancer screening in Spain, France, the U.K. and the Netherlands. It was found necessary to use country specific data on incidence, mortality, demography, screening organization and price levels in health care rather than performing a simple extrapolation of the cost-effectiveness ratio found for the Netherlands. Using this more sophisticated extrapolation technique they found that cost-effectiveness ratios in the U.K., France and Spain were 0.9, 2.7 and 4.6 times as large as the cost-effectiveness ratio for the Netherlands. It is clear that a simple recommendation, based on the Dutch study, to implement nationwide screening in all European countries cannot be sustained. (The issue of transferability of economic appraisal results is discussed in the context of pharmaceuticals in Chapter 5.)

The costs of the economic appraisal and consequent monitoring of the implementation of the screening programme amounted to no more than 1.5% of the total costs of the programme and we would suggest such an investment is cost–effective, provided that the researchers work closely together with the health authorities to make sure that the right questions are addressed and to guarantee that the information from the economic study is correctly interpreted at the policy level and quickly acted upon. This study provides an example where such close cooperation between the research team at the Erasmus University Rotterdam and the secretariat of the Health Insurance Executive Board proved to be very productive.

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5. LIMITING THE REIMBURSEMENT OF TECHNOLOGIES: THE CASE OF PHARMACEUTICALS

5.1 Introduction

Pharmaceuticals are unique among health technologies in that in most countries formal licensing procedures have to be followed before the pharmaceutical company is given approval to market its products. This procedure dates back to the 1960s where there were a number of tragedies relating to the use of products that were subsequently found to be unsafe. (The most notable of these was related to thalidomide, a drug taken by women during pregnancy.)

Therefore, pharmaceuticals now undergo substantial testing for efficacy and safety prior to launch. Although no countries require economic data prior to the registration of drugs, the implication of the licensing procedure for economic evaluation is that there is usually a substantial body of clinical evidence upon which to base economic studies. Thus, in principle it should be easier to undertake good economic evaluations of pharmaceuticals than those of other health technologies.

Another recent development is that two jurisdictions, Australia and Ontario (Canada) have proposed draft guidelines for economic evaluation of pharmaceuticals prior to inclusion on the 'positive list' of drugs reimbursed by the government [Commonwealth of Australia, 1992; Ontario Ministry of Health, 1991]. (The Australian guidelines were implemented in January 1993.) Therefore, in these jurisdictions economic evidence is being enhanced to a status similar to that of

data on efficacy and safety, although the latter remain the prime concern [Drummond, 1992]. The economic evaluation guidelines are symptomatic of the worldwide interest in securing better value for money from health care. However, a major reason why Australia and Ontario have made this proposal is that both jurisdictions already make listing decisions based on the comparative therapeutic usefulness of medicines. Therefore, this is an obvious point at which to introduce consideration of the relative cost–effectiveness of products.

5.2 Decisions About Pharmaceutical Technologies

There are a number of key decision points relating to pharmaceutical technologies. Some European countries operate a national 'positive' list of reimburseable drugs, like Australia and Canada. Therefore, economic evidence could be brought to bear in reimbursement decisions. One example is in The Netherlands, where the Health Insurance Executive Board has, on occasions, asked for cost–effectiveness evidence prior to the reimbursement of new technologies, including some pharmaceuticals [Rutten and van der Linden, 1992]. However, in other countries, like the United Kingdom, all licensed drugs are automatically reimbursed, with a few exceptions in certain therapeutic categories, where a 'limited list' operates. In these countries there is currently not the same opportunity to link economic evaluation to decisions about government reimbursement.

Another major decision concerning pharmaceuticals relates to the price. In the majority of European countries the prices of drugs are the subject of negotiation between the companies and the government. In principle, economic evidence could be taken into account when setting the price of a drug and some national pricing committees have considered economic data submitted by the companies. However, the overall impression is that currently the influence of economic

evaluation results on pricing decisions is very small. Most decisions depend on a number of other factors, such as the investments the company makes on research and its contribution to the local economy.

A more recent development is that of reference pricing. Here the government reimburses not the full cost of each product, but only to a certain level for the therapeutic class. The implication is that where a given product is priced above the reference price, the difference has to be made up by the patient. This approach has been followed both in Germany and The Netherlands. As with pricing decisions more generally, there is clearly scope to consider economic evidence when setting the reference price. This is not normally done, however.

Although a number of key decisions about pharmaceuticals are made centrally, many more are made locally, at the level of the individual hospital or individual prescriber. At the hospital level it is normal to have a formulary of approved drugs, compiled using expert advice. Most formulary decisions are primarily based on the comparative efficacy of products, but comparative cost is also a factor, particularly in therapeutic groups such as anti–infectives where there are a number of products with similar efficacy. Here economic evaluation could play an important role in decision making, as it considers not only drug acquisition cost, but also the costs of the associated medical care and quality of life for the patient.

Influencing the decisions of individual prescribers is more complex, since, by definition, the choice of drug is made on the basis of the individual clinician's judgement. However, in a number of European countries government agencies provide information to prescribers, including information on the comparative cost of products. In some countries, such as the UK and Germany,

there is also control of prescribers' drugs budgets. However, the role of economic evaluation in improving prescribing decisions is currently not well defined, although some pharmaceutical companies have incorporated cost–effectiveness data in their product literature.

5.3 An Example of Economic Evaluation: Drugs for the Management of Labour in Pregnancy

In the management of labour there is a choice between artificial membrane rupture, alone or with oxytocin and prostaglandin E_2 to induce labour. The difference in the acquisition cost of the medicines is clear, approximately £1 (for oxytocin) compared with £20 (for PGE_2) at the dose levels suggested by the literature. Although not a significant extra cost for a given women, the total impact on the pharmacy budget of a hospital may mean that this expenditure comes under close scrutiny.

However, the differing effects of the two drugs may have broader impact on health care costs and the quality of care. Therefore, Davies and Drummond [1991] undertook an economic evaluation considering a wide range of costs, including those of the nursing and medical time in monitoring labour and the costs of managing complications, such as those leading to caesarean section and excessive postpartum haemorrhage.

The economic evaluation was based on a clinical decision tree, shown in Figure 5.1 for the case of a woman with an unripe cervix. It can be seen that, depending on the drug used, there is a given probability of labour starting. These probabilities, and those of spontaneous delivery, caesarian section and post–partum haemorrhage (PPH) were obtained from published controlled clinical trials.

Figure 5.1: Decision Tree: Unripe Cervix

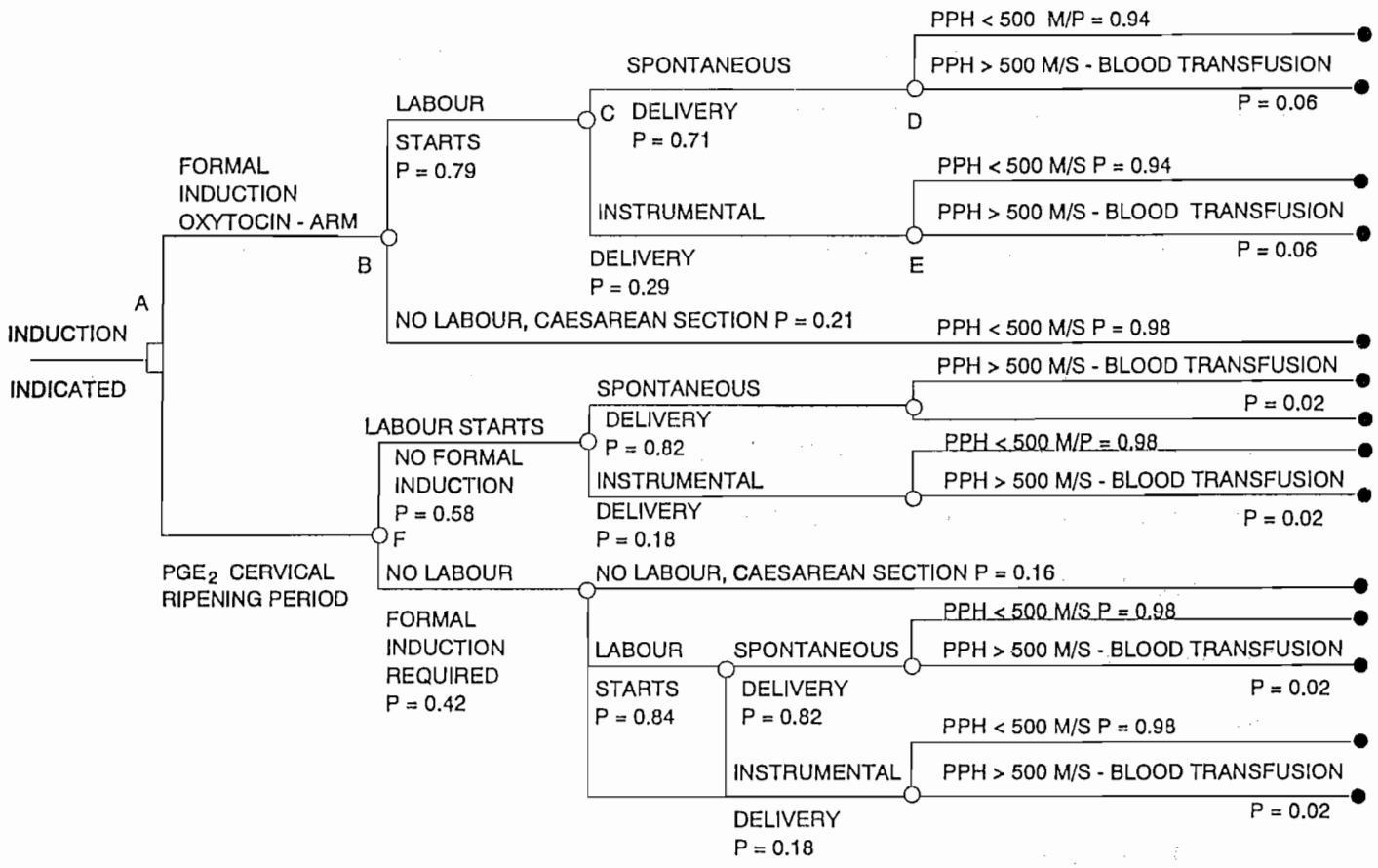

Table 5.1: Expected Costs (in £) per Case of Alternative Strategies

Assumptions	Ripening/induction with prostaglandin E_2	Formal induction with amniotomy and oxytocin
Unripe cervix		
Base case	168	221
Full monitoring costs	247	253
Meta-analysis probabilities	199	208
Partial cost savings from caesarean section	144	148
Ripe cervix		
Base case	95	78
Full monitoring costs	129	100
Meta-analysis probabilities	115	112
Partial cost savings from caesarean section	85	64

Cost data were combined with those on probabilities and the expected cost per case of the alternative strategies calculated under different assumptions (see Table 5.1). It can be seen that, although PGE_2 has a higher acquisition cost than oxytocin, its use leads to an equivalent, or lower, expected cost under most assumptions. For example, for a woman with an unripe cervix the base case analysis suggests that the expected cost per case for PGE_2 is £168, compared with £221 for oxytocin. (This assumed, for example, that the woman was monitored during labour 50% of the time and that the probabilities could be taken from trials of PGE_2.) If full monitoring costs are assumed (i.e. a midwife being present with the woman 100% of the time instead of 50%) the figures are £247 and £253 respectively. If the probabilities from a meta–analysis of all trials of prostaglandins are used, instead of those from trials of PGE_2 alone, then the estimates are £191 and £208 respectively, and so on.

The results of studies like this are potentially of use to decision makers at the hospital level in considering whether or not PGE_2 should be included on the local formulary. Clearly the results need to be interpreted in the light of local factors, since some of the savings (e.g. in medical time in performing caesarian sections) may require managerial decisions to be made in order that they can be realised. There are, in addition, intangible factors, such as the pain of labour, which were not included in the study. In general these favour PGE_2 and would therefore add weight to the argument that it is a cost–effective technology.

The impact of this evaluation has been evaluated by further study [Godman, 1992]. Local decision makers were asked whether they had seen the results and whether these would lead them to modify their actions. Many decision makers replied in the affirmative. Further study has also been conducted in order to assess whether decisions on drug acquisition were actually changed

[Godman, 1993]. Finally, since the original study embodied many assumptions, many of these were updated in a subsequent paper when more data became available. The conclusions of the original study were largely confirmed [Davies and Drummond, 1993].

5.4 International Generalizability of Economic Data

Because there is a growing number of economic evaluations of pharmaceuticals, undertaken in a number of countries, it is possible to address the issue of international generalizability of economic data. This is of interest to decision makers since it is unlikely that every technology could be evaluated in every setting.

This issue is raised in the Australian guidelines where it is suggested that results from elsewhere can be used providing the appropriate adjustments are made. Whereas efficacy data from a given clinical trial may be relevant to other settings, there are a number of factors limiting the broader relevance of economic data. These factors include the demography and epidemiology of disease, clinical practice and conventions, relative price levels, health care resources distribution and availability, and incentives to health professionals and institutions.

A recent paper has explored these issues in the context of the evaluation of a new drug to prevent gastric ulcers in people experiencing symptoms during long–term non–steroidal anti–inflammatory drug use [Drummond et al., 1992]. The same evaluation was performed, using identical methods, in Belgium, France, the UK and the USA. It was found that extrapolation of results from country to country was greatly facilitated if the economic evaluation was structured in a decision–tree format (see Figure 5.2). This enabled data particular to the individual countries to

be introduced in order to examine the impact on estimates of costs and benefits, although the researchers found that data limitations sometimes prevented this being done. Of course, the problem of extrapolation is not limited to international studies since it is well–known that clinical practice and conventions can vary greatly within a given country.

Figure 5.2: **Decision–analytic Model of Prophylactic Use of Misoprostol in Patients with Osteoarthritis and Abdominal Pain who are taking Nonsteroidal Anti–inflammatory Drugs (NSAIDs).**
Adapted from Hillman and Bloom, *Archives of Internal Medicine*, 1989, 149, 2061–65, copyright 1989, American Medical Association.

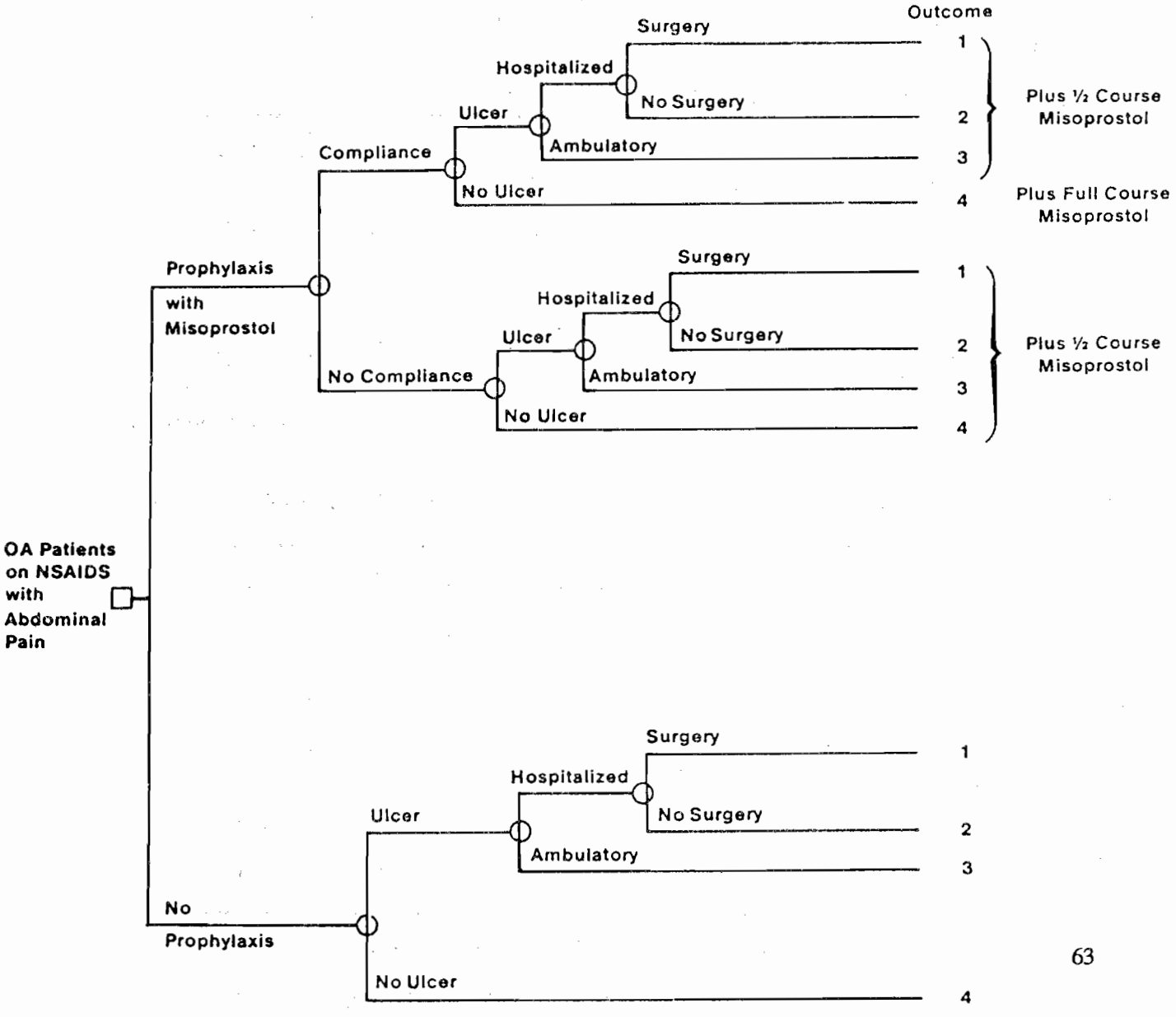

Table 5.2: Input Values: Ambulatory Care

	Belgium	France	U.K.	U.S.
Ambulatory care of ulcer				
Physician (office) visits	3	2	6.4	3
Outpatient (specialty clinic) visits	1.5	0	2.7	0
Endoscopies	1.5	2	1.5	1
X–rays	0	0	0.7	1
H_2 antagonist	7 weeks	6 weeks	6 weeks	12 weeks
*Total cost (in US dollars)**	389	256	540	901
Care of "no ulcer" group				
Physician (office) visits	1.1	2	3.4	2
Outpatient (specialty clinic) visits	0	0	0.1	0
Endoscopies	0.9	0	0.5	0
X–rays	0	0	0.2	0
Antacids	4–5 weeks	6 weeks	6 weeks	0
*Total cost (in US dollars)**	24	48	71	80
Cost of misoprostol (3 months)* (US$)				
800 mcg	132	129	134	180

* Costs are expressed in 1988 prices. Conversions to US dollars from other currencies are made by purchasing power parities [OECD, 1989]. These remain relatively constant through time and are not affected by transient fluctuations in exchange rates.

Table 5.3: Input Values: Hospital Care

	Belgium	France	U.K.	U.S.
Percentage of ulcer patients hospitalized	18.9	6.0	5.3	8.6
Percentage hospitalized patients receiving operation (other than endoscopy)	16.7	19.0	43.2	12.0
Length of hospital stay (days)				
Operation	18	18	9	7
No operation	8	13	10	6
Total cost including follow–up care				
Surgical case (in US dollars)*	4165	6503	2533	15,700
Medical case (in US dollars)*	1793	2569	1548	3450

* Costs are expressed in 1988 prices. Conversions to US dollars from other currencies are made by purchasing power parities.

Table 5.4: Expected Costs (Savings) per Patient for 3 Months of Misoprostol Prophylaxis (in US Dollars)*

	Belgium	France	U.K.	U.S.
High dose (800 mcg daily, 60% compliance**)				
Ulcers 0.3 cm or larger (silent ulcer rate of 40%)	32	61	55	22
Ulcers 0.5cm or larger (silent ulcer rate of 40%)	63	79	72	72
Low dose (400 mcg daily, 72% compliance**)				
Ulcers 0.3 cm or larger (silent ulcer rate of 40%)	5	15	3	(40)
Ulcers 0.5 cm or larger (silent ulcer rate of 40%)	40	35	22	16

* Costs are expressed in 1988 prices. Conversions to US dollars from other currencies are made by purchasing power parities.

** Compliance figures are taken from the study of NSAID–doing regimens. It has also been assumed that 200 mcg twice daily has the same effect as 100 mcg four times daily, the regimen that was tested in the clinical trial.

The results of the international study of misoprostol, the drug of interest, are shown in Tables 5.2, 5.3 and 5.4. It can be seen that, despite great variations in the costs of outpatient care and inpatient care, the overall expected costs of three months prophylaxis are remarkably similar in the four countries studied. However, before many conclusions can be drawn about the international transferability of economic evaluation results, further study is required.

5.5 Pharmaceutical Company Sponsorship of Economic Studies

A recent paper by Hillman et al. [1991] outlined the possible sources of bias in industry–sponsored evaluations. The draft Ontario guidelines suggested that different weight would be given to economic studies, depending on the extent to which the investigators have been given a free hand in conducting and reporting their research. It is further assumed that bias would be less in studies undertaken by independent academic investigators, having an 'arms–length' relationship with the industry, than, for example, in studies undertaken by private consultants with no academic affiliation.

A number of potential biases could occur in both economic evaluations and clinical trials, which are also predominantly funded by the industry. The biases include the choice of question for study, the type of study methodology and the method of reporting the results. It is interesting to speculate why there should be particular concerns about economic evaluations, when the experience with industry funding of clinical trials has generally been good. It could be that economist researchers are regarded as less trustworthy than their clinical counterparts, that unfavourable (to the company) economic analyses are easier to suppress since (unlike clinical

trials) they may not always be visible, or that industrial sponsors are likely to place more pressure on economic analysts than on clinical researchers to produce favourable results.

Although all of the above may be contributory factors, the main reason for extra concern is that economic evaluations, with their less developed methods, may be easier to manipulate. Frequently, they involve the making of assumptions and many evaluations, particularly those based on modelling approaches, have a 'black box' feel about them. Whereas clinical trials are open to manipulation, for example through the use of sub-group analysis, many of the methods are well established, conclusions are reached based on observed data rather than assumptions, statistical tests are performed as a matter of course and the investigators are usually blind to study results until the code is broken.

Therefore, bias in economic evaluations is likely to be minimized if it is viewed by the industry as science, rather than just a marketing ploy. In particular, where a study is intended for publication, the rights of the investigators and the company need to be clearly specified at the outset. However, above all, analysts should use transparent methods that can easily be validated by peer-reviewed journal referees and governmental decision makers. It is much preferable that economic evaluations be assessed in accordance with the methods employed in the study, rather than by merely noting who undertook the study. This means that journal referees and those judging company submissions to governmental committees need to have the skills to tell a good study from a bad one.

5.6 Conclusions

Pharmaceuticals have historically been more subject to evaluation and control than other health technologies. It is therefore not surprising that this is the area of technological development where the formal requirement for economic studies has been first introduced. There has been a rapid increase in the number of economic evaluations of pharmaceuticals, many of which have been funded by the pharmaceutical industry itself. The potential for bias in industry–sponsored evaluations, and the ways to minimize this, were discussed above.

In the past the pharmaceutical industry contributed greatly to the development of clinical trial methodology. If carefully managed, the industry's interest in economic evaluation may yield similar benefits. At any rate, it is likely that the economic evaluation of pharmaceuticals will be one of the most developed, both in terms of method and of application of economic evidence in decision making.

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6. PLANNING SPECIALIST SERVICES: THE CASE OF HEART TRANSPLANTATION

6.1 Introduction

In principle, the planning of specialist facilities offers an excellent opportunity for the use of economic appraisal in determining the rational diffusion and use of health technologies. First, specialist facilities often represent 'big ticket' items, so there is a prima facie case for taking decisions about them with care. Secondly, because the development of specialist facilities often requires official approval, for capital expenditure or a licence to provide the facility, there is an opportunity for the government (or other decision making agency) to regulate by directive [Haan and Rutten, 1987].

Finally, there are good clinical reasons for not allowing a proliferation of specialist facilities. That is, there is a 'learning curve' for specialised medical procedures and there are thus strong arguments in favour of concentrating them in a few locations, where a strong clinical team can be assembled by having the opportunity to perform a number of such procedures. This means that there is likely to be slightly lower clinical resistance to the application of economic criteria in decision making about specialist services, than in situations where there is a strong belief among clinicians that technologies should diffuse more widely.

Potentially, there are a number of decisions about the planning of specialist facilities that could be subject to economic appraisal. These include: which specialized facilities to provide, how many to provide, where to locate them and their appropriate size. A number of studies have been

carried out, including those of open heart surgery units, lithotripters, megavolt therapy, and magnetic resonance imagers.

Another feature of decisions about specialist facilities is that, in general, the major decision is only taken once for a given location. Therefore, of necessity, economic appraisal of investments in specialist facilities are often undertaken using only minimal local information and often draw heavily on data for appraisals undertaken elsewhere.

Therefore this Chapter, while being concerned with heart transplantation, also explores a methodological issue. Namely, to what extent can the results of an economic appraisal undertaken in one location be adapted for use in a second location? It discusses, first, an economic appraisal of heart transplantation undertaken in the UK for 1982–1985 and then a subsequent study, undertaken in the Netherlands [de Charro et al., 1988; van Hout et al., 1993]. Several general lessons are drawn concerning the problems and prospects of economic appraisals undertaken to inform decisions about specialist facilities.

6.2 Economic Evaluation of Heart Transplantation in the United Kingdom

6.2.1 The decision making context

In the United Kingdom decisions about the development of specialist services like heart transplantation typically involve the Department of Health or Regional Health Authority since central funds are required. By 1982 around 50 heart transplants had been performed over the previous two years in two centres – Papworth and Harefield Hospitals. In the future the

programmes at each centre were then expected to involve 15 to 20 transplants per year. A review of funding arrangements was required since, despite public support, the programmes relied heavily on a flow of charitable donations. Also, the possibility of funding a third centre had been discussed.

Therefore in 1982 the Office of the Chief Scientist at the Department of Health commissioned an economic evaluation of the two existing programmes from a team consisting of researchers from Brunel University and University of Cambridge. The study was published as a Department of Health monograph in 1985 [Buxton et al., 1985] and considered programme costs, survival and quality of life of patients.

The results of the study have been fully discussed elsewhere [Buxton et al., 1985]. The purpose here is to outline some of the major methodological issues raised by the study and the uses to which the study results were put. (These points are also discussed at greater length in Buxton [1987].)

6.2.2 Methodological issues

One major methodological issue arose from the fact that, for ethical reasons, patients could not be randomized to study and control groups. Therefore, quality of life improvements were based on a 'before and after' assessment. The gain in survival was estimated using data from historical controls and from patients whom were unable to be transplanted owing to the lack of a suitable matching organ.

Another major methodological issue, affecting the estimation of both costs and benefits, was the changing nature of the technology. Some major changes, such as the replacement of the immuno-suppressive regimen by cyclosporin A, were easy to detect and to adjust for. Others, such as a gradual decline in the length of patient stay, were harder to identify and to interpret. In order to deal with changes over time the analysts adopted an approach whereby observations on patients were grouped in terms of standard six-month periods (or 'cross-sectional views') from the date of their transplant in order to build up a composite picture of post-transplant costs (see Buxton [1987] for more details).

More generally the study raised the issue of the appropriate timing for such an evaluation of an emerging technology. The analysts recognized that there was probably no right time for the study; an evaluation could be 'too early', in that developments in expertise in the future might improve outcomes and reduce costs. On the other hand an evaluation could be 'too late', in that the technology may be well-established before the results of the evaluation were known. More recently, it has been argued [Banta and Thacker, 1990] that evaluations of health technologies should not be viewed 'one-time' exercises and that they need to be approached on an iterative basis.

6.2.3 Results of the study

The transplant programmes were found to increase both survival and quality of life. The main question for economic evaluation was 'at what cost?'. The costs considered were all those associated with patients' in-patient stays at, and out-patient visits to, the transplant centres, subject to the exclusion of salaries of the surgical team, which were treated as overheads to the

programmes, and capital costs. In addition, patient–specific estimates of the full drug costs were included irrespective of from where the drugs were dispensed. The average costs for the first six months (including the cost of the transplant operation itself and the immediate in–patient care) was £12,370 per patient at Harefield and £14,960 per patient at Papworth.

However, the average costs masked some important differences in cost structure between the two centres. Tables 6.1 and 6.2 show (respectively) the average costs per patient for assessment and from operation to discharge. The analysts pointed out that consideration of these data could greatly add to our understanding of the potential to generalize economic evaluation results from the two centres studied and the potential for improvements in the cost–effectiveness of the programme as a whole. For example, at Harefield considerable savings were made in nursing costs by accommodating the patients in apartments (flats) on the hospital site, rather than in hospital wards, during periods where their condition needed to be monitored but where intensive nursing support was not necessarily required.

The results of the study were reported in a disaggregated form, giving data on costs, quality of life and survival separately. On balance the analysts thought this preferable to a more speculative analysis, aggregating the results in terms of the incremental cost per quality–adjusted life–year gained (see Chapter 2 above). However, they did recognize that, in reaching policy decisions about the funding of alternative health technologies competing for the same resources, good summative information is necessary.

Table 6.1: Average costs per patient (£, 1983/84 prices): assessment (1 July 1983 to 30 June 1984) (Source: Buxton et al. [1985].)

Resource item	Assessment	
	Harefield (n = 65)	Papworth (n = 49)
Nursing	390	83
Consumables	39	8
Drugs	33	7
Respiratory physiology	25	2
Radiography, etc.	64	12
Pathology, etc.	181	120
Electrocardiography	20	1
Other	11	27
Sub–total	763	260
General services	418	169
GRAND TOTAL	1181	429

Table 6.2: Average cost per patient (£, 1983/84 prices): operation to discharge (1 July 1983 to 30 June 1984) (Source: Buxton et al. [1985].)

	Transplant operation to discharge		
	Harefield (n = 47)		Papworth (n = 21)
Resource item	Hospital	Flats	
Nursing	1490	–	2735
Consumables	149	–	273
Drugs	767	452	1445
Recipient operation	934	–	1711
Respiratory physiology	47	–	–
Radiography, etc.	170	68	584
Pathology, etc.	642	153	1656
Blood products	377	–	465
Electrocardiography	66	17	76
Physiotherapy	26	–	110
Sub-total	4668	690	9055
General services	207	207	2103
GRAND TOTAL	4875	898	11158

6.3 Adaptation of the Original Study for the Netherlands

6.3.1 The decision making context in the Netherlands

The first Dutch heart transplant was performed on June 23, 1984 without formal approval of the health authorities. After this event the authorities decided quickly to license two transplant centres to perform a limited number of heart transplants and to initiate a technology assessment of introducing heart transplantation in the Netherlands, making use of the experience in the two transplant centres in Rotterdam and Utrecht. This was in accordance with the strategy of the Health Insurance Executive Board to support decisions on major new health care interventions with information from economic appraisals. The decision making context of the Dutch study was quite similar to that of the study in the U.K. Questions to be answered were:

- is heart transplantation a cost–effective intervention that should be funded out of public resources?

- if so, how many centres are to receive a license to perform a heart transplant programme?

- given the obvious restrictions on such programmes (e.g. the limited number of donor organs) how many people would receive a heart transplant and what would be the costs of such programme in the long run?

As a technology assessment of a heart transplant programme had been carried out before both in the U.K. and in the United States [Evans, 1984], there was the additional question whether or not to initiate a Dutch heart transplant study. A specific Dutch study was thought to be necessary because estimates of the costs in the U.K. and U.S.A. showed large variation, and because the costs and effects of the alternative (of no transplant programme) needed more analysis. Also, the technology was already further developed than in the days of the earlier studies and finally, the long term implications of including heart transplantation in the Dutch health care system had to be investigated [van Hout et al., 1993].

6.3.2 Methodological issues

As in the U.K. the major methodological problem was the lack of a suitable control group. Estimates of costs and effects without a heart transplant programme had to be based therefore on data from patients who were actually referred to a heart transplant centre. From these data the situation without a heart transplant programme had to be constructed. Another major problem was the necessity to estimate the long term survival of those with a heart transplant, as the actual observation during follow-up was limited to a maximum of about 1250 days. And finally, forecasts had to be made of the future numbers of patients and the future numbers of donor organs and the associated costs and effects of the programme. In this case micro simulation was chosen as the appropriate technique to take account of the heterogeneity in the patient population and because of its power to model and visualize the interaction between number of patients referred to the waiting list, numbers of donor organs and survival probabilities [van Hout et al., 1993].

Although the strategy of the Health Insurance Executive Board was to initiate an assessment of all relevant aspects of a particular technology, in this case the evaluative team of the Erasmus University Rotterdam was only asked to consider the cost–effectiveness of the programme. One of the major ethical questions in this case was, of course, that given the limited availability of donor organs there has to be some rationing procedure for accepting a person in the programme and for selecting persons on the waiting list for receiving a transplant. In the course of the assessment it became clear that no more than half of the patients indicated would eventually receive a transplant. As this issue of rationing was debated heavily after the results of the assessment became available, an opportunity was missed to include this issue in the assessment study and to include, for instance, an analysis of whether or not there should be a specific and explicit procedure for rationing and for possible appeals from patients who are not satisfied with the decision making process.

6.3.3 The results of the study

Unlike the English study there was no detailed analysis of the differences between costs in the centres in Rotterdam and Utrecht. Costs per life year gained for the whole programme were estimated at DFL 57,650 and costs per quality adjusted life year gained were estimated at DFL 71,900. Table 6.3 from van Hout et al. [1993] shows the results of a sensitivity analysis, where the effects, on the estimated costs per life year gained, of changes in some of the major parameters are shown.

Table 6.3: Sensitivity Analysis; Changing the Base Line Estimates by + and -20%

variable	base line estimate	CHANGE IN COSTS PER LIFE YEAR GAINED (C_{lyg}) (Base line estimate - 57650 DFL) new estimates C_{lyg} (-20% - +20%)	percentage change (-20% +20%)
'Net' costs of screening per transplanted patient	28,942	(56,840 - 58,460)	-1.41% 1.40%
Costs of treatment on the waiting list per patient per year:			
with transplant programme	63,134	(56,450 - 58,849)	-2.08% 2.08%
without transplant programme	52,676	(60,654 - 54,645)	5.21% -5.21%
Costs during the first year after transplant	132,156	(54,071 - 61,228)	-6.21% 6.21%
costs during later years after transplant	37,396	(49,655 - 65,644)	-13.87% 13.87%
survival on the waiting list	400 days	(55,228 - 59,869)	-4.20% 3.85%
survival after 1 year after transplant	10.98 year	(62,342 - 54,797)	8.14% -4.95%
proportion transplanted	0.52	(58,938 - 56,856)	2.23% -1.38%
discount rate	0.05	(56,382 - 59,029)	-2.20% 2.39%

Source: van Hout et al. [1993]

The table shows that the cost-effectiveness ratio is sensitive to the costs during later years after transplant (medicines). A special effort was made to measure quality of life before and after transplant. This revealed that there is a considerable improvement in quality of life in this patient group.

The U.K. and Dutch investigators together analyzed the differences in costs for the transplant programmes in both countries [van Hout et al., 1992]. Table 6.4 shows the costs during the first year including transplantation in the U.K. and the Netherlands, in US $ adjusted by purchasing power parities (PPPs).

Table 6.4: Costs During the First Year after Transplantation: the U.K. and the Netherlands

	The United Kingdom (1984 US $ ppp)			The Netherlands (1987 US $ ppp)		
	Volume	Price	COSTS	Volume	Price	COSTS
donor operation	1	1691	1691	1	1738	1738
transplant operation	1	902	902	1	4165	4165
inpatient days	47	224	10511	44	235	10247
outpatient visits	18	24	435	27	57	1556
x–rays	72	9	627	35	23	803
biopsies	13	47	600	15	367	5512
catheterisations	0.37	142	53	2	798	1348
ecg's	34	9	329	44	20	889
t–cells assays	28	27	733	42	13	555
cyclo–assays	34	9	329	48	13	635
physiotherapy (hours)	12	13	151	11	33	376
gates bloodpool scans	1	9	9	3	111	308
drugs			9702			12702
laboratory tests			3529			7485
other services			813			4089
Sub Total			30414			52408
OVERHEAD						
staff time			74455			97348
administration			–			34091
social work			6809			53719
outpatient nurses			14029			49587
capital costs			–			278926

Source: van Hout et al. [1992]

One of the problems in making these comparisons is the choice of common denominator of costs. In this case medical purchasing power parities were chosen, which can be found from OECD statistics. From the table we conclude that the programme in the Netherlands is much more expensive than that in the U.K. Indeed, if we would assume a similar programme with similar resource input in natural units, such programme would cost 70% more in the Netherlands than in the U.K. when corrected for general differences in purchasing power in both countries [van Hout et al., 1993].

6.4 Uses of the Study Results

6.4.1 United Kingdom

The United Kingdom heart transplant study is often cited as a good example of an economic appraisal that had an impact on policy. Certainly the Department of Health did consider these data, alongside other factors, when arriving at its decision to continue funding of heart transplants at these centres and at other centres in the future. It may be that the data only confirmed prior prejudices that heart transplants should be funded. Certainly the decision suggested by the economic appraisal – to continue funding – was politically easier to take than that of discontinuing funding.

Although the UK heart transplant study is highly regarded, both for its methodological quality and its practical relevance to decision making, Buxton [1987] points out that there are often changing expectations, on the part of decision makers, about such evaluations. He stresses that, although the agreed research protocol adopted a method appropriate to answering a number of

limited questions, at different points of time various relevant parties hoped, or expected, that the study would provide answers to many different questions, each of which ideally required a slightly different study; namely:

How much do the transplant programmes cost the hospitals at which they are based?

What impact do they have on the other work–load of the hospitals?

Which of the two centres is the most cost–effective?

Do patients survive longer after transplantation than they otherwise would?

Should central funding continue to the existing two units?

What is the optimal scale of activity?

What should be the location of a third (or subsequent) centre?

How does heart transplantation currently compare with other heart procedures in terms of value for money?

How does heart transplantation compare with heart disease prevention programmes in terms of value for money?

How does heart transplantation compare with other quite unrelated uses of health services resources in terms of value for money?

What will be the picture in five years time?

Given such a plethora of questions, it is vital that the economic appraisal and its presentation should provide a robust information and data base for a variety of subsequent possible analyses.

6.4.2 The Netherlands

Also in the Netherlands the study can be said to have had a definite impact on policy making. The cost-effectiveness ratio resulting from the study was thought to be acceptable, but the decision to include heart transplantation in the public insurance package was postponed until late 1990. There was ample discussion about the necessity for rationing (as discussed above) and furthermore, there was some lack of consensus about a protocol that included an age limit of 55 years. As it is known that heart transplantation may be beneficial for selected patients above 55 years of age [Miller et al., 1988], the age limit was called discriminatory on "non-medical" grounds and the Health Council advised the Minister not to agree to this age limit. van Hout et al. [1993] argue that this is an interesting case of a trade-off between efficiency and equity, as the study showed that the additional costs of screening and treatment of patients above 55 years will exceed NLG 3,000.000 per year. Because of the limited number of donor organs, this figure can be set against almost no beneficial effects. In this case the equity argument was favoured and a formal age limit was not accepted.

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7. CONCLUSIONS

The purpose of this handbook was to illustrate the problems and prospects for using economic appraisal as part of a policy to encourage a rational diffusion and use of health technologies. This has been done by considering three case studies, selected from a much wider range of economic appraisals identified by the Network established by the EC Concerted Action [Davies et al., 1993]. In this final section we consider what has been learned from the case studies themselves and the more general prospects for the role of economic appraisal in decision making.

7.1 Screening Programmes

The case study of screening for breast cancer concerned a national decision whether or not to initiate a programme. Being a central decision it was possible to control the development of the programme and to determine an evaluation strategy. In the Netherlands this included pilot projects, which were useful in confirming some data from previous studies overseas and in generating new data pertinent to the economic appraisal.

The economic appraisal required computer models and simulations to be developed. This was necessary because of the long term effects of the programme, which stretched far beyond the time period that could be studied. However, a major advantage of this approach is its flexibility and its ability to produce data pertinent to new issues as these arose (e.g. the impact of improving attendance rates).

A particularly interesting feature of this study was its ability to inform decisions about the organizational aspects of the programme (such as the development of mobile units). The iterative nature of the appraisal proved very useful for decision makers. The analysis was also updated after the programme had been in operation for a period of time; this identified situations where costs or effects had been different from expectations and the potential for cost savings.

The evaluations also showed that it is not possible to take results from other countries without question. It also addressed the question of value for money from performing economic appraisals.

7.2 Pharmaceuticals

The case study on pharmaceuticals dealt with a situation where there are many decision points about the technology and a multitude of decision makers (e.g. central government, hospital, individual prescribers). Therefore, in examining the role of economic appraisal it is important to specify these decision points and to consider where it would be possible, in a given country, to influence decision making.

This case study also illustrates that economic appraisal is greatly assisted when clinical evidence is available. Therefore there has been considerable growth in the economic appraisal of pharmaceuticals and the studies are often performed to a good standard. The majority of studies are being funded by the industry itself and some concerns have been raised about this. However, ways can be found to minimize potential bias in evaluations.

The evaluations performed of pharmaceuticals have also used decision analytic models that are amenable to computerization. This enables the analysis to be re-worked where there are uncertainties about some of the data and it also facilitates the transfer of economic appraisal results from one setting to another. This case study illustrated how the results of studies could be easily adapted by specifying a model in advance and by adding limited quantities of local data.

The case of pharmaceuticals is also unique in that it is the first where formal economic appraisal requirements have been specified by government. The outcomes of these initiatives will only become apparent later. However, the mere act of specifying requirements has caused both government and industry to think more about evaluation methods and has made apparent the need for government decision makers to have access to the necessary skills for assessing studies.

7.3 Specialist Services

The case study of heart transplants illustrates some of the potential and the difficulties of undertaking economic appraisals of 'big ticket' technologies. Because of the expense involved in setting up these services, there are natural limitations on their diffusion. However, in both the countries studied it was necessary for government to intervene to limit the spread of transplants and to insist on their evaluation.

The main technical problems addressed in the studies themselves were those of rapidly changing technology, the absence of an adequate control group and the lack of data.

This case also illustrates that, when major evaluations are being planned, there are often many stakeholders having quite different questions. It is impossible for a single evaluation to answer every conceivable question. Finally, this case study provides an example where economic analysts in the different countries were able to collaborate, with assistance from the Concerted Action, to maximize the learning from the appraisals conducted [van Hout et al., 1992].

7.4 General Lessons for Policy Makers

Based on these case studies and the other outputs from the Concerted Action, what are the general lessons for those policy makers wishing to use economic appraisal in encouraging a rational diffusion and use of health technology?

(i) The methods are not perfect but they are good enough

The methodology of economic appraisal is continually developing and many issues remain unresolved. However, the case studies illustrate that it is possible to generate data that can improve decision making.

(ii) There is no shortage of available policy instruments

Decision makers often despair about their lack of ability to influence the diffusion and use of health technologies. These studies have illustrated that there is a wide range of options available. The secret is in choosing the best policy instrument from the range. This will differ from one technology to another.

(iii) The analysis needs to be integrated with the decision making process

The case studies illustrate that there is much to be gained from targeting the analysis to answer specific questions posed by those charged with making decisions about health technologies. A continuous dialogue between analysts and decision makers is useful. In some cases the decision maker may commission the evaluation directly, as in the case of the evaluation of specialist services. In other cases, such as pharmaceuticals, government may specify guidelines for undertaking studies.

It is also apparent that, as technologies develop and diffuse, the policy issues may change. Therefore it is important to view these evaluations as an iterative process and not as a 'one shot' exercise.

(iv) The analysis needs to be relevant to local circumstances

Given the need for analysis to be well focussed, it is not easy to use results from studies performed elsewhere. This issue was addressed in all three case studies in slightly different ways. We believe that methods are now available to help in the transfer of economic appraisal results, although this is not straightforward. Therefore, there is a continuing need for international collaboration in specifying methodological standards and in technology policy development. However, the most productive studies for decision making will continue to be those using local data to address local questions.

7.5 Taking Matters Further

This handbook gives only an introduction to the use of economic appraisal in decision making about health technologies. Decision makers within the EC wishing to take this further should consult a forthcoming special issue of the journal Social Science and Medicine. This reports the proceedings of a recent EC workshop, where these issues were discussed in more details and where recent developments in most member states were outlined.

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