The OECD Health Project

Health Technology and Decision Making

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Foreword

The OECD initiated the Health Project in 2001 to address some of the key challenges policy makers face in improving the performance of their countries' health systems. A desire for real progress and recognition of important gaps in the information needed to undertake change led to political commitment and support across countries for a focused cross-national effort. The three-year initiative provided member countries with multiple opportunities to participate in and learn from component studies focused on pressing health policy issues. Countries also benefited from the information and exchanges that occurred, first at the kick-off conference in Ottawa, Canada in November 2001, and at no fewer than 20 subsequent meetings of officials and experts in venues ranging from Paris to The Hague to New York.

Performance improvement requires grappling with difficult questions. What can be done to ensure that spending on health is affordable today and sustainable tomorrow? What is needed to improve the quality and safety of health care, and to ensure that health systems are responsive to the needs of patients and other stakeholders? How should equitable and timely access to necessary care be supported? And perhaps the most challenging question of all: what can be done to increase value for money?

The Health Project offered a means for officials in member countries to learn from each others' experiences in tackling these questions, to draw upon the best expertise available across OECD member countries and within the OECD Secretariat, and to break new ground to support health-system performance improvement in the future. It encompassed nearly a dozen studies addressing key policy issues pertaining to human resources in health care, new and emerging health technologies, long-term care, private health insurance, health-care cost control, equity of access across income groups, waiting times for elective surgery, and other topics that are central to the policy concerns of OECD member countries. It was not possible to address every issue important to Health Ministries in the course of the Project, but the issues that were chosen were ones considered to be of the most pressing importance.

The Health Project built on the foundation of the OECD's work in health statistics and health policy that has been carried out under the purview of various committees and working parties across the OECD. An important contributor to the success of the Health Project was its horizontal approach. Work in progress was discussed by experts and Delegate groups with a variety of important perspectives on health policy issues. The Project benefited from the guidance and support of an Ad Hoc Group on Health, made up of Delegates from member countries, and the specialised expertise of various OECD directorates was employed in tackling issues. The Directorate for Employment, Labour and Social Affairs took the lead in co-ordinating the work conducted in horizontal co-operation with the Economics Department, the Directorate for Science, Technology and Industry, and the Directorate for Financial and Enterprise Affairs.

From my own political experience, I know how significant the results of this project will be for policy makers at the most senior levels of government. There are no governments within the OECD or beyond which will not derive important benefits from this work as they all struggle to meet varying challenges in the field of health care. It is apparent that there are few one-off solutions or quick fixes. But this project has demonstrated that benchmarking within and across countries, and sharing information can bring new ideas together and help policy makers meet those challenges.

Donald J. Johnston
OECD Secretary-General

Preface

This report presents the findings from the OECD Project on Health Technologies. This project was part of the OECD Health Project, under the guidance of the Ad Hoc Group on Health. The project and final report has greatly benefited from the support and comments received from experts during a series of meetings held between April 2002 and March 2004. The expert meetings were chaired by Dr. Jane Cook, Dr. Bernie Towler and Mr. Damian Coburn from the Australian Department of Health and Ageing. The list of expert meeting participants can be found in Annex 1.

The project's preliminary results and analysis were presented at a workshop held in The Hague, the Netherlands on 27-28 October 2003. The OECD would like to thank all workshop participants for their contributions (the programme can be found in Annex 2), as well as the governments of the Netherlands and Australia for their support in co-sponsoring the workshop. The OECD is particularly grateful to Mr. Jeroen Hulleman and his colleagues at the Dutch Ministry of Health, Welfare and Sport for their assistance in organising the workshop.

Mr. Kees van Gool and Dr. Iain Gillespie from the OECD Secretariat have contributed to Chapters 1, 3 and 4 of this report. Chapter 2 draws on two consultant reports that were drafted for this project. Chapters 5 to 8 were written by lead countries authors and consultants, and their names appear at start of these chapters. Chapter 9 draws on the report prepared by Professor John Gabbay in his capacity as workshop rapporteur.

Finally, the authors would like to thank Mr. Dean Huisman for assistance in survey design, Dr. Philip Haywood for research assistance, Dr. Gillian Sykes for drafting comments, Mr. Joseph Loux for preparing the publication and Ms. Stella Horsin, Ms. Lorna Wilson, Ms. Diana Morales and Ms. Fabienne Michel for secretarial assistance.

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EXECUTIVE SUMMARY

The introduction of new technologies has brought remarkable improvements over the past few decades to the health of the citizens of OECD member countries. There is, however, widespread variation in how such new, as well as existing, technologies are used across OECD countries, indicating that the most effective and efficient technologies may not always be the ones employed. Encouraging the uptake of the most efficient and effective health-care technologies remains a significant policy challenge in many OECD countries.

The work described in this report, carried out with a group of experts drawn from OECD countries, examined how improvements could be made to integrate effective and efficient technologies into health-care systems. Analysis focused on the production of evidence, primarily in the form of health technology assessment[1] (HTA), and the way that such evidence is subsequently used in decision making. The way decisions are made and implemented was also analysed, including examining the range of policy tools used to put decisions into actual practice.

The conclusions presented in this report drew on analysis of a survey of how health technologies are integrated into health systems, focusing on:

- How decisions at the national, regional and hospital levels are made.
- How evidence (particularly HTA-based evidence) is produced and used in decision making.
- How aspects of health-care systems facilitate or impede the implementation of decisions.

Twelve OECD member countries[2] participated in the survey and respondents included government officials, health technology assessors, health care and hospital administrators, clinicians and researchers. Comparative information was collected on actual decision-making processes for five case study technologies: positron emission tomography, hepatitis C genotyping and viral load testing, telemedicine, prostrate cancer screening, and technologies for dealing with stroke patients.

The report considers in depth three of the key challenges that policy makers face when reaching decisions about the use of new technologies. Namely, options for dealing with uncertainty in the evidence base, consideration of the transferability of evaluations between different situations, and analysis of how biomedicines challenge decision makers. Finally, the practicalities of using HTA in three OECD countries are explored.

1. HTA has been developed to consider the broader impacts of health technologies and evaluate their benefits and costs. It typically involves: *i)* identifying the policy question, *ii)* systematic retrieval of scientific evidence and analysis, and *iii)* appraisal of evidence, including judgements about the meaning of the evidence. The evidence and its appraisal then inform the decision-making process.

2. Australia, Austria, Canada, France, Germany, Ireland, Japan, Mexico, the Netherlands, Norway, Spain and Switzerland.

The production and use of HTA-based evidence

The process of health technology assessment (HTA) is widely regarded as able to deliver succinct, high quality and trusted evidence to decision makers at all levels of the health-care system and many OECD countries are developing and implementing policies to support the production and improvement of the range of approaches and methodologies involved. However, investment in developing the field still tends only to reflect fractions of a percent of total health-care spending and HTA continues to face significant challenges. In particular, although the standard of evidence produced by HTA is generally regarded as high, thus far there is only limited evidence of the effectiveness of HTA in terms of its influence on decision making, on health technology use or on health outcomes.

A range of initiatives are underway in the countries sampled by the survey to improve communication of the results of formal assessment to a broad range of decision makers and stakeholders, such as policy makers, clinicians, industry and patients. In most cases, these efforts are primarily focused on dissemination. Whilst there remains no definitive consensus on what dissemination methods work best, nor whether what works well in one setting will work equally well in another, there is some evidence from the survey that a "portfolio" approach may be more effective than any single approach to disseminating the results of HTA analysis. Significant efforts have been made in some OECD countries to develop a "culture" of evidence based policy and practice by stimulating activities that generate greater demand for HTA, including education programmes targeted at developing decision makers' skills in interpreting and analysing evidence, establishing information infrastructure to make evidence more readily available and developing decision-making processes with a more clearly defined role for HTA.

Health technology assessments are more likely to be used by decision makers if policy instruments are available to act on the assessment, if prior commitments to make use of technology assessments are in place, and if the technology assessment methodology used is regarded as "appropriate". The OECD survey results suggest that technology assessment largely needs to be tailor-made for the characteristics of individual health systems, including where decisions about uptake and diffusion of technology get made as well as, in some cases, for the particular technologies being assessed. In general, HTA needs to be linked more comprehensively with innovation and other aspects of policy making.

For some technologies, the pace of development tests the capacity of HTA producers to keep assessments current. HTA producers generally only occasionally undertake assessment updates to keep abreast of research and development, citing lack of resources as the main barrier. Where they have been done, more forward looking assessments have had some impact by highlighting areas of uncertainty, suggesting areas for further research, and outlining potential areas for further improvement of technologies: in some cases these kinds of assessments have helped define producer-purchaser agreements for specific technologies. Efforts have been made by some health service providers to access more timely information for decision making through the use of foresight-type exercises (such as horizon-scanning) to identify possible future technologies, although as yet there is little evidence of the actual impact of such approaches on subsequent decision making.

Despite these challenges to health technology assessment, the OECD survey found that there is widespread support from decision makers for the continuing provision of HTA. This is borne out by several recent reviews of HTA in, for example, the United Kingdom and Denmark.

The decision-making process

Access to high-quality evidence is necessary but not sufficient to ensure rational uptake and diffusion of health technologies. It is increasingly recognised that the rational use of evidence depends, in large part, on the decision making process and on the institutional, organisational, political and cultural dynamics of health care systems.

For each of the case-study technologies examined, decisions about the uptake and diffusion of health technologies take place at multiple levels of the health care system. Where clear decision-making structures existed, the impact of HTA evidence on decisions seemed to improve, perhaps because HTA practitioners were better able to direct their assessments to pre-defined target groups.

There is evidence of growing use of "conditional approvals" for the funding/reimbursement of new technologies by health service providers. These can provide opportunities to grant access to technologies where this might not otherwise be the case, while collecting further data to overcome uncertainty and minimise potential risks. However, for conditional approvals to be effective, there needs to be clarity between stakeholders about what data will be collected by whom as well as how and when it will be used to confirm final approval or otherwise. There is some concern that too liberal use of conditional approvals could inappropriately slow access to new technologies. This could potentially undermine the existing system and the impact of conditional approvals thus should be monitored.

In general, decisions about health technologies are more likely to be accepted by a broad range of stakeholders, such as health professionals, industry and patient representatives, if the decision making process is regarded as transparent and based on evidence, and if it includes an appeals mechanism. Stakeholder acceptance of decisions seems to be a key determinant of whether decisions are actually put into practice within health systems.

Better dialogue early on in the assessment process is required between the producers and users of HTA to achieve better alignment of HTA content, decision makers' needs and policy.

Implementing decisions

The success or otherwise of HTA needs to be addressed primarily in terms of the downstream impact on health outcomes and the performance of health systems. Certainly, many OECD countries are gearing their health systems towards an era in health policy that places stronger emphasises on measurement, accountability, value for money and evidence-based policies and practices. The challenge for many policy makers is to develop policy tools that aim to achieve multiple health system goals simultaneously and particularly to develop methodologies that elaborate how evidence-based decisions might translate into direct impacts on health outcomes.

Currently, increasing attention seems to be given to presenting evidence-based decisions in such a way that they are more likely to be given effect in actual practice. Three main factors seem to be involved. First, the process by which results are presented and disseminated. There is a significant heterogeneity here, though the OECD survey suggested that the development of clinical guidelines or recommendations and the development of specific health programmes are the most common approaches used (at least in the countries sampled).

Second, there appears to be growing understanding that the institutional and financial aspects of the health system have a crucial influence on whether decisions will be successfully implemented. Additional funding and flexibility between budgets were important facilitators in implementing decisions. Health system characteristics, including provider payment mechanisms, can create strong (dis)incentives to efficient decision making, leading to potential under use of cost-effective technologies.

And third, the level of stakeholder trust in the evidence, and in the systems that use it, emerges as a key determining factor. One means to help engender such trust appears to be the principle of risk-sharing. A number of health service providers in OECD countries have started to use novel techniques, such as agreements with technology providers and industry, to implement decisions that aim to capture the benefits of new technologies, help overcome uncertainties and recognise the value of true innovation, within the constraints of overall health system resources. These techniques require more rigorous evaluation but may offer greater promise to deliver on the multiple health system objectives that many OECD countries are aiming for.

Decision making under uncertainty

Uncertainty remains pervasive in decision making about new health technologies yet decisions nevertheless have to be taken, since no decision, or a severely delayed decision, is effectively a decision not to supply and/or reimburse. Two main strategies for managing uncertainty seem to be in use. First, creating closer links between technical experts and policy makers and, second, applying risk-amelioration strategies.

For decisions that occur at national or state-wide levels, the problems of applying technical information to policy in a considered and policy-relevant way can be reduced if there are formal linkages between users and producers of HTA. Formal HTA liaison or management infrastructure within ministries can foster knowledge and expertise within the administrative arms of governments. Such expertise can help administrators to recognise uncertainty, avoid reliance on simplistic solutions, and develop policy measures that might deal with uncertainty more effectively.

As already noted, a number of examples exist of risk-sharing agreements between industry and governments or other health service providers to help deal with uncertainty. These include price-volume agreements and/or commitments to gathering further data. Such agreements need to have clearly defined parameters understood by both parties, with clear end points and appropriate opportunities for review.

Transferability of economic evidence

Decision makers often face difficulties in determining whether the results of economic evaluations, which may be carried out elsewhere, are relevant to their own local circumstances. This is partly due to the frequent lack of transparency in the reporting of such evaluations, but even when the knowledge base concerning a particular clinical intervention is robust, the extent to which specific evidence, rather than evaluations, can best be taken account of in decisions may depend on local conditions.

Four main issues arise. First, there can be significant lags between technological developments and health-care evaluations. To some extent these may be ameliorated by risk-sharing agreements but these may be insufficient in themselves. There is therefore a need to strengthen the analytical enterprise for evaluation, perhaps through new public and private partnerships that enable the costs of health evaluations to be shared.

Second, equity and distributive justice considerations need to be taken account of. These may vary according to the technology or indication in question as well as according to the prevailing local ethical or social policy environment. Including wider stakeholders (patients, health-care providers and public representatives) can help bring wider perspectives to the appraisal and decision-making process.

Third, there remains uncertainty over the extent to which evidence can be transferred from setting to setting. There seems to be general recognition that appraisal decisions are generally setting-specific but a lack of transparency in reporting means that it is often difficult to assess the relevance of economic evaluations to their local setting, or to extrapolate the results. The development of clear shared reporting frameworks could ease this situation.

And finally, there is little evidence so far that economic evaluations do well in determining which subgroups of potential patient populations might benefit most from an intervention. This will become a bigger challenge as pharmacogenetics-based products come online.

Biotechnology and decision making

Biotechnology is seen as being capable of providing significant positive change in delivering better health. However, a number of factors about the technology bring particular challenges to the way economic evidence currently is generated and used in decision making. These relate mainly to speed of development of biotechnology, investment risks and returns, potential high cost and high effectiveness, and ethical as well as public perception considerations.

Decision makers seem likely increasingly to require more evidence on all aspects of medical innovations, not just the clinical and economic effect but also ethical, social and macroeconomic characteristics, particularly as new health-related biotechnologies come on stream. The question is whether HTA can or should adapt its methodologies to capture these issues, or whether such evidence should come from other sources.

More empirical work is required to open the “black box” of decision making to see better how policy makers use the various tools and evidence available to them. Some methodological refinement of HTA is required, for example in how to use pharmacogenetic data, whereas other issues, integration of ethical considerations, for example, may more appropriately fall outside HTA.

There is thus a need to explore possible policy frameworks within which such broader issues can be addressed. The absence of a clear policy framework to deliver much needed information to decision makers can create significant uncertainty for innovators. The development of such a framework focused on reduction of risk for innovators as well as the integration of the broader policy considerations in decision making on biotechnologies seems necessary if the potential health and economic benefits of the technology are to be maximised. The lessons learned from such an exercise might be applicable also to other technologies that exhibit similar complex characteristics.

New biomedical advances have thrown into sharp relief the need for a better dialogue over the right questions to ask about technologies, and the right mechanisms – technical and political – for answering them. This in turn highlights the need for more appropriate training and development for assessors and decision makers and much closer links with technology innovators.

National experiences with HTA

The use of HTA in decision making requires access to trusted evidence that is relevant to the decision maker's needs. Experiences from three OECD countries highlight how this challenge is being addressed within the context of the organisational and institutional frameworks of their respective health care systems.

In **Australia,** the production and use of HTA at the federal level have developed to meet the needs of two health care financing programmes covering pharmaceuticals and privately provided medical services. This has resulted in HTA activities that are focused on policy makers' requirements. However, HTA has been more limited in terms of its influence on clinical practice and areas beyond the reach of the two federal programmes. Recent efforts have concentrated on delivering HTA to a broader set of decision making processes.

In **Canada**, HTA activities have devolved along provincial lines and have been co-ordinated through the Canadian Coordinating Office of HTA (CCOHTA). Recent efforts have focused on developing a comprehensive HTA strategy that considers the impact of technologies and maximizes their effectiveness, as well as increasing the capacity and utility of HTA. National efforts are underway to recognise and promote health technology assessment as a valuable and necessary tool in health decisions, and to invest in co-ordination and collaboration within the country, which is seen as essential to ensuring more efficient production of HTA as well as its use in decision making.

In **Mexico**'s complex health system, co-ordinating HTA activities and facilitating their use in decision making remains a major undertaking although recent efforts have shown promise. To promote the use of HTA, the Centre for Technological Excellence was recently established to develop national policies for HTA. Meanwhile, the Mexican Institute of Social Security-IMSS (the main public body responsible for providing health care delivery to almost 50 million Mexicans) is in the process of developing mechanisms to facilitate the use of HTA at all levels of health care decision making. Work on the effective integration and co-ordination of HTA in decision making has only recently commenced but has already seen some success. The challenge ahead for Mexico is for HTA to reach a wider audience of decision makers.

Experiences from Australia, Canada and Mexico demonstrate that to be useful to decision makers, HTA must be tailored to individual characteristics of the health-care system, including consideration of where decisions about uptake and diffusion of technologies get made. Greater collaboration between the producers and users of HTA – and with technology providers, is seen as a key success factor in integrating HTA into the health care system in a way that delivers better health system performance and health outcomes.

Chapter 1

INTRODUCTION

Health-care technologies have contributed to the extension of human life and to the reduction of pain, disease risk, and disability. However, there is widespread variation in the uptake and diffusion of health-care technology amongst OECD countries, indicating that there are opportunities for more effective integration of such technologies into the health system. This introductory chapter sets out the objectives and methodology for the OECD project on health technologies. It also presents readers with the concepts and definitions used throughout the book.

Health care is an important economic, scientific and social endeavour for all OECD countries. It has contributed to the extension of human life and reduction of pain, disease risk, and disability. It is broadly recognised that the application of new knowledge and technological change is a key driver of these achievements. For example, research shows that the rate of technological change in OECD countries is positively related to health outcomes and quality of life for patients who have suffered heart attacks (Atella, 2003).

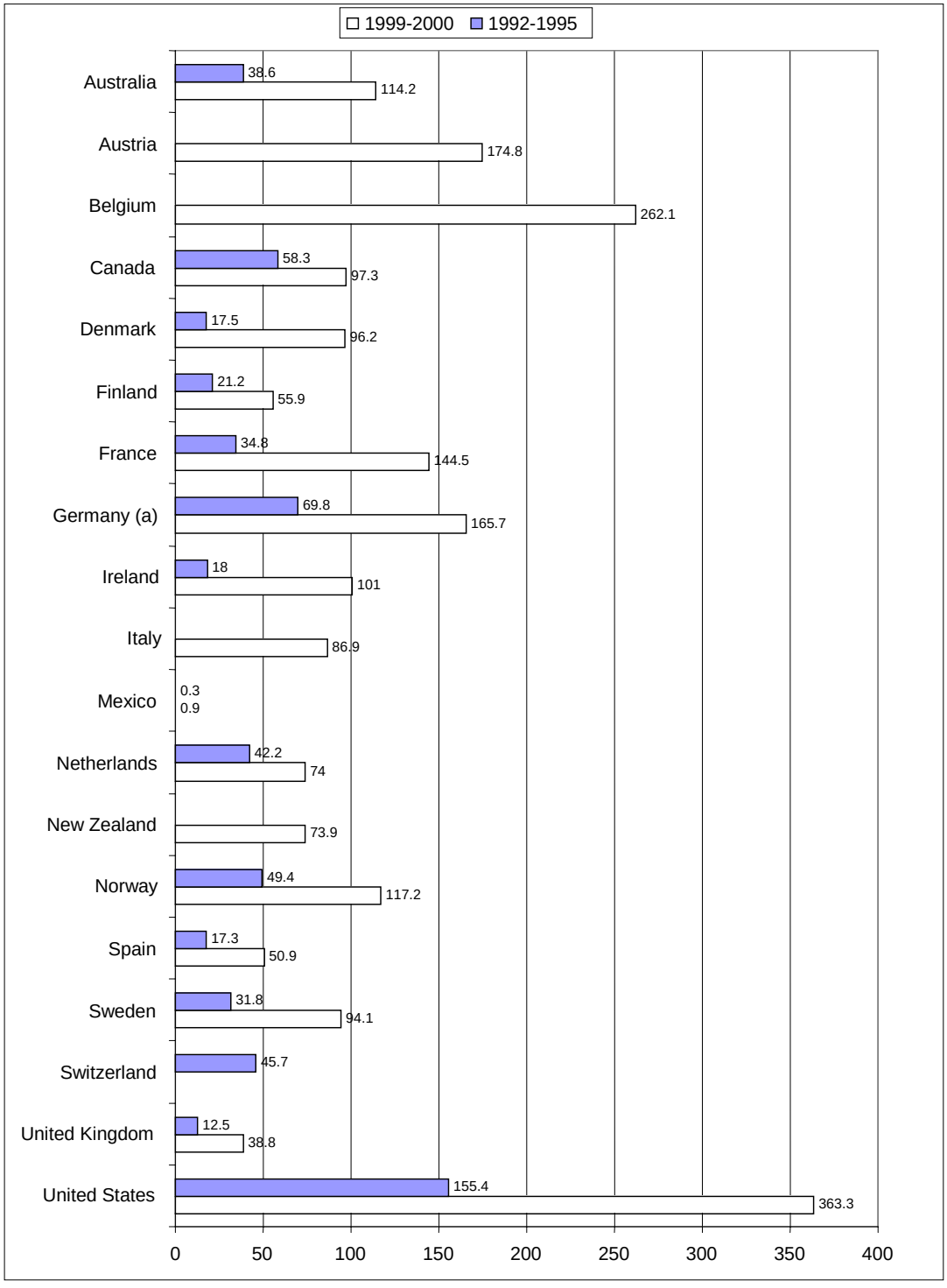
However, in recent decades health-related activities have been consuming growing proportions of GDP. In 1990, the average rate of health-care spending in OECD countries was 7.3% of GDP. By 2001, this average had risen to 8.4%, representing an increase of 15% over and above GDP growth (OECD, 2003). In the context of lower economic growth, the ageing of the population and the rise in health-care costs, many OECD governments are concerned about how to ensure the sustainability of public health-care financing.

Technology is seen as a driver of health costs. Studies by Aaron (1991), Jones (2002) and Newhouse (1992) report that as much as 50% of total health-care spending growth can be attributed to technological change. Furthermore, a study by Fuchs (1996) indicates that this proposition has become a dominant view amongst health economists. However, other economists argue that "direct evidence on the role of technological change in cost growth is lacking" (Cutler and McClellan, 1996)[1].

A further empirical observation is that there is widespread variation in technology utilisation and diffusion across and within OECD countries. Figure 1.1, to give but one example, shows the number of percutaneous coronary interventions (PTCA and stenting) per 100 000 population in 19 OECD countries between 1992-95 and 1999-2000. The average number of procedures per 100 000 population for those 19 countries was 41 between 1992 and 1995. This number increased to 118 by 1999-2000. The level of variation can be seen in Figure 1.1, which shows that in 1999-2000 the number of procedures in Mexico was 0.9, whereas the figure for the United States was 363.

1. The same authors found in a later study that the total benefits of technological change exceeded the corresponding costs for at least four of the five conditions over the period studied (findings for breast cancer were equivocal) (Cutler and McClellan, 2001).

Figure 1.1. **Number of percutaneous coronary interventions (balloon angioplasty and stenting) per 100 000 population, 1992-95 and 1999-2000**



(a) Figures date from 1992 and 1997.

Source: OECD Health Data 2003.

Variation in uptake and diffusion can signify the sub-optimal use of technology. It could signify that some countries are "overusing" the technology and other countries are "underusing" it. For instance, a technology is "overused" when the costs outweigh the benefits for any additional level of technology diffusion or use. "Underuse", on the other hand, can occur when the forgone benefits outweigh the costs of additional diffusion or use. Both scenarios are sub-optimal and can bring economic costs and/or reduced health outcomes. It is important to note however that not all technology variation necessarily indicates "overuse" or "underuse".

There is in fact a multitude of reasons that can explain the variation in technology diffusion, including:

- *Variation in health-care needs*. For example, countries may have different disease prevalence rates and therefore the variation in technology use might be a reflection of differing health needs.
- *Economic conditions*. For instance, there is considerable evidence that supports the theory that richer countries are in general willing to spend greater proportions of their GDP on health care (and technology).
- *Cultural and organisational features of the health-care system*. For example, the reimbursement mechanisms and the incentives that health-care providers and institutions face can contribute to their willingness to purchase and use new health technologies.
- *National regulations*, which, despite continuing efforts at harmonisation, can still vary considerably.

Decision making in the health-care system: assessment, appraisal and implementation

Making better decisions about the uptake and diffusion of health technologies is an issue of increasing concern to policy makers. Better educated health consumers, providers of health services, a large scale international health industry, media reporting and advertising may create expectations that health technologies will become available in a timely (or even instantaneous) way. This often includes an expectation of public funding. Consequently, a range of expectations put pressure on policy makers and health system decision makers in a way that is sometimes characterised as the "technological imperative".

The challenge for many policy makers is to create policies that can harness the benefits of technology and innovation, but at the same time achieve multiple health system objectives within the constraints of fiscal policy.

Since the 1970s, OECD countries have increasingly recognised the value of health-care decision making within an evidence-based paradigm. Such evidence enables informed choices to be made about the diffusion and use of new and emerging health technologies to prevent, treat and manage disease. In the absence of clear evidence, the uptake and diffusion of technologies are more likely to be influenced by a whole range of social, financial, professional and institutional factors, and may not produce optimum levels of health outcomes or efficiency. To this end, many OECD countries have invested in the production of evidence, frequently using health technology assessment (HTA) to consider the broader impacts of health technologies and evaluate their benefits and costs.

HTA has been described as "the bridge between evidence and policy making" (Battista & Hodge, 1999). It provides information for health-care decision makers who are involved in funding, planning, purchasing and investment decisions.

The HTA process comprises three steps:

- Identification of questions, including the prioritisation of the topic (for example what is the additional benefit of technology "X" over technology "Y" in diagnosis of disease "Z"?, or "what is the optimal strategy for management of disease Z?") and development of a strategy to answer these questions.
- Systematic retrieval of scientific evidence and analysis, critical review and summary of the evidence, including comment on the validity and strength of the evidence[2].
- Appraisal of evidence, including judgements about the meaning of the evidence obtained by systematic review and the formation of views as to the value of a technology in the health-care system. The evidence and its appraisal then inform the decision-making process.[3]

The way HTA is produced varies considerably amongst countries. Some countries have national assessment agencies that conduct or co-ordinate all HTA activities, whereas other countries have more devolved responsibilities for producing HTA. At a minimum, HTA addresses the efficacy of technologies, including: *1)* the health benefits to patients, *2)* potential side effects and *3)* comparisons (of health benefit) with alternative technologies. Broader HTA will frequently include economic evaluation, typically in the form of cost-effectiveness analysis (CEA). There is widespread agreement amongst HTA agencies on the definition of HTA. A review of some of the major agencies and guidelines reveals that the definition always includes references to effectiveness and cost-effectiveness and also mentions the examination of broader consequences of health technologies such as the legal, ethical, psychological and social implications. There is, however, considerable variation in the way HTA is practised and integrated into OECD member country health-care systems.

Whilst HTA is still a relatively new concept, it is a rapidly growing field. The US Congressional Office of Health Technology Assessment was a pioneer in the field, making significant contributions to the concept of HTA in the mid seventies and producing major reports on, for example, computed tomography in 1978. In 1987, the Swedish Council on Technology assessment in Health Care (SBU) was established, and in 1988 the Dutch established the Investigational Medicine Fund (Banta, 2003). In the United Kingdom, 2003 marked the ten-year anniversary of HTA in the National Health Service (NHS).

Until now there has been little systematic evidence on the impact HTA has had on decision makers, or on decision implementation. Two recent reports state that concrete examples of the impact of health technology assessment were hard to find and that the

2. Though it is strictly not a 'review', this component of HTA may also include modelling, especially to support economic analysis.

3. This specific division is not universally applied; some would place decision making as part of the HTA process, and in some circumstances appraisal and decision-making may be conducted by the same people. The important point here is that review, appraisal and decision are conceptually distinct parts of an HTA-supported process.

evidence base on how to translate evidence into practice is very limited (ECHTA, 2002; Maynard, 2000). Little is known about the many factors that can deliver improved use of HTA in decision making and better integration of health technologies in the health-care system, although there is some evidence that a portfolio of dissemination strategies might help reach a greater number of health-care decision makers (Lehoux *et al*., 2003).

However, access to high-quality evidence is widely recognised as a necessary, though not sufficient, requirement to manage the uptake and use of health technologies effectively. The decision-making process itself is increasingly recognised as an important part of successfully using evidence and implementing recommendations reached through evidence-based assessment.

There is an identified need to look at decision making as a whole in the health-care system, to gain a better understanding of its processes and the use of evidence. Better knowledge of decision-making processes will contribute to a better understanding of the widespread variation in technology diffusion and utilisation.

The OECD's project on health-related technologies

The OECD's project on health related technologies was part of the three-year OECD Health Project. It was overseen by the OECD's Ad Hoc Group on Health and was guided by delegates (from here on referred to as the Expert Group), drawn together from government, academia, health technology assessors and industry. They met on five occasions, between April 2002 and March 2004. The names of participants can be found in Annex 1.

This OECD project aimed to provide evidence on how countries can improve the integration of technology into the health-care system. Its focus was on the decision-making process and the policy tools used to implement decisions, as well as the transition from HTA to decision making. It examined the production of HTA and the use of evidence in decision making.

Figure 1.2 illustrates the conceptual decision-making process inside health-care systems which was used in this project. It places decision making at the centre of the system; important inputs into the decision-making process include HTA and evidence on the impact of new technologies, along with the appraisal of evidence and other factors such as economic conditions, organisational features, and national and international regulations.

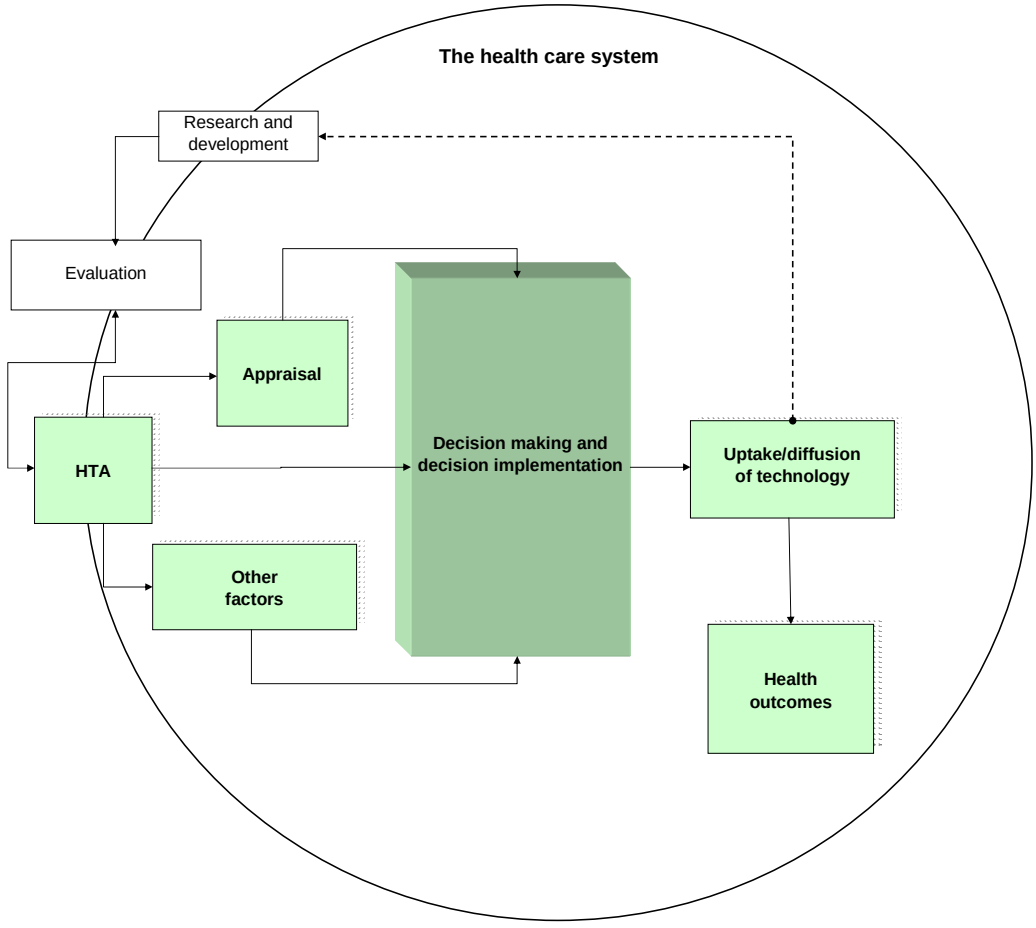
With the required inputs in hand, decision makers then determine whether and how to adopt and implement the new technology. For example, policy makers may decide to add a new technology to national reimbursement schemes on the basis of decision inputs that suggest it represents good value for money. The decision-making process and the method of implementing decisions can impact on the uptake and diffusion patterns for health technologies and, in turn, health outcomes.

Figure 1.2 provides a generic view of the health-care decision-making process, but of course there are large variations within this model. The historical development and evolution of health-care systems play an important role in setting the institutional characteristics of the decision-making processes. For example, OECD member countries place different emphasis on the incorporation of HTA in the decision-making process, with some countries making it compulsory and others making it voluntary. Who makes the decision is also likely to vary. In some countries, decisions are made at a national

level while in other countries the same decision might be made at a regional level. For example, the decision to reimburse a new technology can be made at national or provincial government levels and also at private health insurance levels.

Figure 1.2 indicates that some activities such as research, development and health service evaluation can take place across national borders. Furthermore, health technology assessments can (and do) use international research results and sometimes assessments are conducted jointly amongst a number of HTA agencies. Finally, Figure 1.2 links technology uptake and diffusion with the research and development that take place. This link is created by the various signals (including price and volume) sent from the health-care system to the research and development community.

Figure 1.2. **Outline of HTA, decision making and implementation**

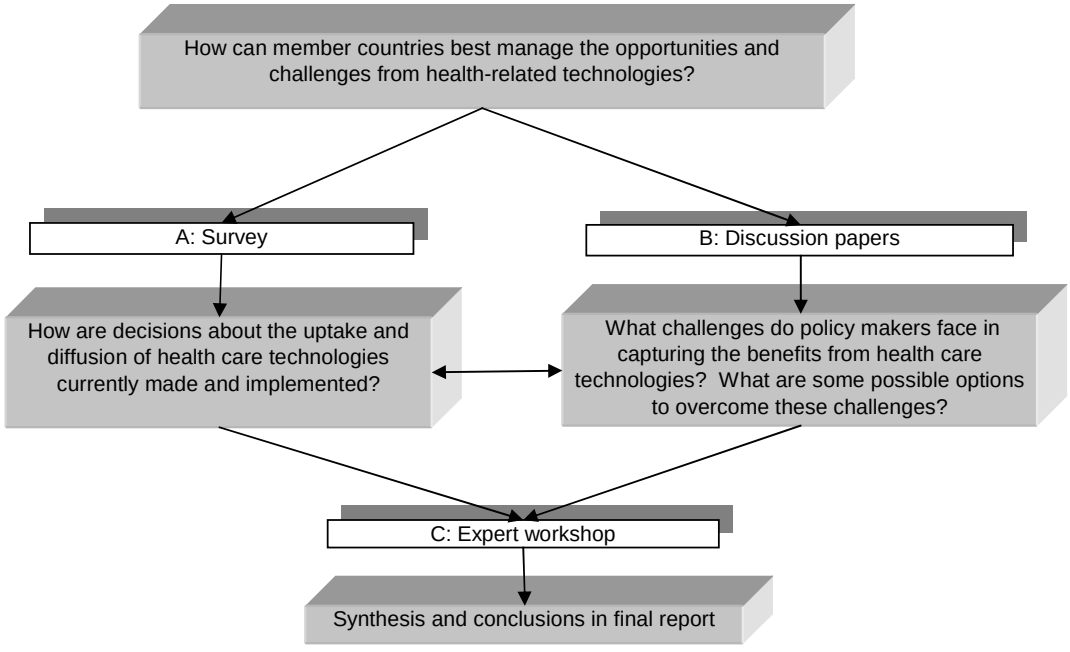


The OECD project was divided into three parts, as illustrated in Figure 1.3. Part A of the project sought to survey current practice in decision making and implementation, and the use of HTA; Part B considered the key challenges in decision making and the use of HTA; and part C synthesised and discussed the results of parts A and B in an expert workshop. These three parts of the OECD Health Technology Project have provided the materials for this report.

Part A: Develop, implement and analyse a survey[4] on how OECD countries integrate health technologies. The survey focused on past and present decision-making processes and the production of HTA, seeking information on:

- How decisions about health technologies are made.
- How evidence, and in particular HTA, was used in that decision.
- How HTA was conducted.
- How decisions were implemented within health-care systems.

Figure 1.3. **Structure of the OECD project on health technologies**



Decision making in any health-care system is a complex set of interactions amongst a wide array of players. However, it is possible to categorise the different types of decisions made in different levels of the health-care system. In broad terms, decisions can be categorised into three levels:

- Macro (decisions made at national, provincial or insurance company level).
- Meso (decisions made at regional health authority or hospital level).
- Micro (decisions made at provider or patient level).

The survey focused on macro and meso levels of decision making, and also examined how those decisions were implemented to influence micro level decisions. In particular, it looked at *1)* pre-marketing decisions: the decision to allow a product/service to be used in the health-care system; *2)* funding/coverage decisions: the decision to fund/cover a product or service on either private or public insurance schemes; and *3)* investing/ planning: the decision to invest in a technology, including health-care planning decisions.

4. A summary of survey questions can be found in Annex 3.

The survey analysis primarily focused on funding/coverage and investing/planning decisions.

To enable collection of comparative information, the Expert Group developed and agreed on the methodology for studying five case study technologies as part of the OECD survey. These were: positron emission tomography, hepatitis C genotyping and viral load testing, telemedicine, prostate cancer screening and stroke technologies. These technologies were selected by the Expert Group on the basis of an agreed set of criteria that aimed to ensure the case studies would reflect the diversity of uses of health-care technologies (*e.g.* use of technology in screening, diagnosis, treatment, and management) and settings (*e.g.* ambulatory care, hospital care). Together, the set of case study technologies illustrate decision making in various parts of the health-care system.

Twelve countries participated in the survey: Australia, Austria, Canada, France, Germany, Ireland, Japan, Mexico, the Netherlands, Norway, Spain and Switzerland. Survey respondents were identified by members of the Expert Group and nominated on the basis of their expertise in either the decision-making process and/or the production of HTA for any of the five case study technologies. Survey respondents included departmental officials, health technology assessors, health-care and hospital administrators, clinicians and researchers.

Part B: Whereas part A of the project examined current decision-making processes, part B focused on a number of broader challenges to decision making and HTA. The four key challenges identified by the Expert Group were:

- Decision making under uncertainty.
- Transferring the results from evaluations between countries.
- Facilitating the use of evidence in different OECD health-care systems.
- Policy decision making in the field of new health-related biotechnologies.

For each of these issues, Expert Group members developed draft discussion papers[5] that take a forward looking approach to describe and anticipate key challenges to assessment and decision making, as well as offer some conclusions and potential recommendations. The discussion papers subsequently benefited from discussions at the workshop (part C, discussed below) and consultations amongst the expert group.

Part C: An international experts' workshop was held to bring together the available evidence from the project. Along with expert presentations, the workshop heard and discussed the interim results of the survey and draft discussion papers. The workshop was held in The Hague on 27-28 October 2003 and was co-sponsored by the Dutch Ministry of Health, Welfare and Sport and the Australian Department of Health and Ageing.

Workshop participants played an essential role by discussing and interpreting the project's results and placing these within the national contexts of OECD countries. To supplement the project's findings, the workshop also heard from world experts in the field of health technology assessment and decision making. A list of workshop participants can be found in Annex 2 of this report.

5. Delegates from lead countries produced three of these papers and the fourth paper was produced by external consultants.

Report outline

Chapter 2 of this report provides a literature review on what is known about the dynamics of health technology innovation and the mechanisms that affect health technology uptake and diffusion. It also critically examines policy tools used to help manage the integration of technology into the health-care system. This chapter draws on two papers that were drafted by consultants for this project.

Chapters 3 and 4 present results from the OECD survey on health technologies. Chapter 3 focuses on the production of HTA in survey countries and Chapter 4 examines decision-making processes and implementation methods in survey countries. More detailed comparative survey results and analysis are provided for two case study technologies (PET and stroke technologies). These two case study technologies comprehensively illustrate the high level of HTA activities in survey countries and also demonstrate the variability in the way decisions are made and implemented.

Chapters 5 to 8 present analysis of the broader challenges to decision making and HTA based on expert discussion papers. Chapter 5 provides case studies on how three OECD countries are coming to terms with creating a health-care system environment that is conducive to the incorporation of evidence in decision making. Chapter 6 identifies the potential pitfalls that decision makers face in transferring results from economic evaluations from one setting to another. It sets out three possible areas for policy consideration that may help deal with these pitfalls. Chapter 7 examines uncertainty in decision making and provides some possible processes that can help manage uncertainty. Chapter 8 takes a critical look at whether policy makers are equipped with the right set of tools to deal with the opportunities and challenges that new biomedical advances present.

Chapter 9 offers some concluding observations and sets out the policy implications from the OECD project on health technologies. It identifies the strengths and weaknesses of the project and outlines some important gaps in current knowledge where future OECD work might be warranted.

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Chapter 2

INNOVATION AND INTEGRATION OF HEALTH TECHNOLOGIES: A REVIEW OF THE ISSUES AND LITERATURE

Medical innovation does not just appear. There are important interactions between OECD health-care systems and R&D that shape the speed and direction of medical advances. This chapter provides a literature review on the dynamics of health technology innovation and the mechanisms that affect health technology uptake and diffusion. It also critically examines the literature related to the policy tools used in a number of OECD countries to help manage the integration of technology into the health-care system.

Introduction

Technology[1] is a driver of better health outcomes. Throughout the 20th century technological change has brought new forms of medical care that contribute to the extension of human life and reduction of pain, disease risk, and disability. At the same time, technologies are a major driver of expenditures and the pattern of technology diffusion shows that the effective and efficient technologies are not always adopted most quickly (Greer, 1988; Cowan and Berkowitz, 1996; Davidson, 1995). It is therefore not surprising that policy makers feel a sharpening imperative to manage the social, health and economic impacts of new and emerging technologies

Efficiently integrating technology into health-care systems is complex and difficult. Many thousands of decision makers collectively influence technology diffusion and uptake and decisions on technology diffusion translate directly into coverage decisions — who gets what medical care, on what terms — that bring these difficult issues into the public gaze.

Furthermore, medicine is a highly innovative field. A barometer of technological change is the approval of new drugs and devices by the United States Food and Drug Administration (FDA). In 2002, the US FDA approved some 78 new drug applications and 152 expanded indications for existing technologies. The FDA also approved 34 major new biological agents and 34 biotech agents that were substantially equivalent to existing products as well as 4 949 new or modified devices, including 41 major new devices. This is in addition to many advances in clinical procedures not reflected in the activities of the FDA. But invention is one thing, adoption another: the fruits of medical progress do not appear in final and definitive form on the physician's doorstep.

This chapter considers the dynamics of medical innovations and identifies the key determinants of technological change in the health-care system. It examines the evidence on the strengths and weaknesses of policy tools used to manage technological change,

1. In this context, technology is to be interpreted broadly as encompassing "drugs, devices, medical or surgical procedures used in medical care as well as the organisation and supportive systems within which such care is provided" (Juzwishin *et al*., 1996).

including an examination of health technology assessment (HTA) and cost effectiveness analysis (CEA). The analysis takes into account the many health system policy objectives that OECD member countries share. This chapter draws from two consultant papers. The first of these was written by Dr. Clive Pritchard, Office of Health Economics, and the second by Annetine Gelijns, Lawrence Brown, Daniel Heitjan and Alan Moskowitz from Columbia University.

The dynamics of medical innovation

To understand the economic consequences of technological change, one needs to explore the "mechanisms of action" by which technologies affect health-care costs, and the overall outcomes associated with their use (Gelijns, 1994). The first part of this dynamic is the introduction of new or modified technologies into practice. Policy makers need to know whether the new things that are constantly being invented substitute for or are "add-ons" to existing diagnostic and treatment approaches. They will also want to know whether these technologies are cost-reducing, cost-neutral, or cost-effective, and what the target population is: what kind of volume can we expect?

Technology does not just spring up out of the laboratory; it is shaped by the demand conveyed by its purchasers. In the past, the characteristics of the health-care market sustained an environment in which price considerations played a much smaller role in the adoption of new technologies than they did in non-medical fields. As a result, judgements by the relevant medical specialities about the technologies' clinical performance predominantly set the direction in which improvements were sought. Feedback signals from users to the R&D sector often emphasised shortcomings in efficacy, safety, and ease of operation, and there was little convergence with national health priorities or a need for cost reduction. More recently, however, these signals have been changing. The growing importance of economic considerations in hospital purchasing and clinical adoption decisions explicitly rewards cost reducing technologies or, at least, technologies with a reasonable cost-effectiveness ratio.

The following sections look at the "mechanisms of innovation" in the fields of diagnostics, pharmaceuticals, procedures and devices. They also examine the health and economic impacts of expanding indications once innovations have been introduced into the health-care system.

Diagnostic technology innovation

Two diagnostic areas that continue to show rapid technological change are imaging and the biological markers of disease.

Imaging has progressed from simple planar images to 3-D representations of body anatomy that also offers insight into physiological activity. We can expect that advances in imaging devices, including high-speed computed tomographic (CT) scanning (*e.g.* for lung cancer), ultrasound, magnetic resonance imaging (MRIs), and positron emission tomography (PET) scanners, will continue well into the future. These advances in imaging have aimed to minimise morbidity, maximise the information gained, and reduce costs through faster image production and improved quality.

Advances in mapping the human, and other, genomes have yielded new genetics based technologies that delineate patients with the same indication into those who will and will not benefit from treatment. Such technological development aims to detect diseases at the cellular level, before they become grossly or radiographically evident, and

to predict outcomes. What is the impact of these diagnostic advances on the demand for health-care services, and thus on the economics of health care? One can distinguish three categories.

First, earlier identification of disease may prevent manifestation or delay progression of disease. Such advances, which may delay or prevent serious illness, have the potential to reduce health-care utilisation and thereby cut the costs of treatment.

The second category is diagnostic technologies that may outpace our prognostic or therapeutic knowledge. When diagnostic advances lead to the earlier detection of disease and are not coupled with advances in treatment, the overall proportion of the total population identified as having disease will increase, as also will the demand for further diagnostic tests and treatments, many of which may be ineffective or inefficient. For example, prostate specific antigen (PSA) tests can potentially detect prostate cancer screening for those with early stages of disease. However, there is less conclusive evidence that treatments for prostate cancer such as radiation and surgery are life prolonging. Furthermore, the associated "side effects" of treatment such as impotence and incontinence can be devastating and expensive (Olsen and Gotzsche, 2001; Horton, 2001; Barry, 2000). The lack of consensus on screening and treatment has led to significant variations in treatment patterns for prostate cancer between the United States and Europe: Americans more frequently turn to further testing, prostatectomy, or radiation than do some Europeans (Waymont, 1993).

A third category emerges from technologies that offer more precise information than current diagnostic tools and that can subsequently be used in altering treatment patterns. In breast cancer, for example, women are often treated with radiation and chemotherapy (that often include substantial risks and side effects) to decrease the occurrence of secondary tumours, even though an estimated 70-90% of women will not suffer from recurrence and might do fine without such therapies. However, the introduction of gene based diagnostics might more accurately predict which women are more likely to suffer recurrence, and therefore predict who might benefit from such therapy and who would not (He, 2001). If this technology could be replicated in large trials, this change would obviously reduce the cost of care for this cohort of patients, over and above the benefits to individuals of averting unnecessary adverse events.

Pharmaceutical innovation

Traditional pharmaceutical products have tended to be targeted at symptomatic manifestations of disease. Cholesterol lowering drugs and anti-inflammatory agents have been shown to be effective in modifying the development of vascular disease, both as primary and secondary preventive measures, and are now mainstays of therapy for coronary artery, cerebro-vascular, and peripheral vascular disease. By and large these therapies are expensive and need to be used over the long term, but they have the potential to reduce disability and therefore the use of other health-care services. A recent study, in fact, suggests that the use of many newer drugs tends to reduce the frequency and duration of hospitalisations, resulting in substantial reduction in costs in other parts of the health-care system (Lichtenberg, 2001).

Pharmaceutical development has grown more concerned with developing pharmacologic alternatives to widely practised procedures such as coronary artery bypass surgery, transurethral prostatectomies, and cholecystectomies. Pharmacological alternatives for such procedures have become preferred R&D targets for pharmaceutical manufacturers, as have finding solutions for costly chronic care (*e.g.* Alzheimer's disease).

With advances in molecular biology, drugs can become better tailored to individual patients. Such newer classes of drugs are being developed to delay or prevent the onset of degenerative or progressive diseases, such as Alzheimer's disease or rheumatoid arthritis. Such innovations are designed to increase an individual's likelihood of responding to the drug as well as reduce the risk of side-effects, but there may be some policy challenges around privacy and discrimination (see Chapter 8).

Future developments in the fields of personalised medicines and gene based diagnostics (referred to above) may lead to some convergence of the diagnostic and pharmaceutical markets. While such developments may bring health benefits, they are also likely to lead to a number of policy challenges concerning approval mechanisms, service delivery changes and funding mechanisms.

Innovation in procedures and devices

The mortality and morbidity associated with surgical interventions have dropped considerably over the years. Organ transplantations may be expensive, but generally they are very cost-effective, and natural restrictions on their supply make them a low cost item in the aggregate health-care budget. However, much innovation aims to assist failing organs or replace them with artificial devices. Recently, left ventricular assist devices (LVAD) have been shown to markedly improve one-year survival, as compared with medical management, and offer improved quality of life as well (Rose, 2001). While these technologies may prove to be cost-effective (studies on this are currently in progress), the size of the chronic heart failure population could still render them a sizeable budget item. Hip replacements and other orthopaedic appliances offer no impact on survival rates but substantially affect quality of life and functional capacity. By maintaining an individual's mobility and autonomy, they could reduce the use of other health-care services. For example, a recent Canadian HTA found that stroke patients with mild or moderate disability who receive organised care in a stroke unit are more likely to be alive, independent and living at home after a stroke than those who receive care in a general ward. The study also found some evidence of modestly lower costs (Noorani *et al.*, 2003).

A major trend in traditional surgery and other areas of clinical intervention is the movement toward minimal access therapy. Technological developments in optics, miniaturisation, and electronics are transforming many open, direct visualisation surgical procedures into minimal access, indirect visualisation interventions. While these developments often decrease the per-unit cost, they can also lead to an expansion of demand: both healthier and sicker patients may opt for the therapy once the procedure has become less risky and uncomfortable.

The economic impact of expanding indications

The economic impact of technology depends on how the medical profession influence the use of these technologies after their introduction, and how their indications of use expand. Research has shown that the use of existing products (*e.g.* pharmaceuticals) for new indications is widespread, and accounts for up to 40% of total sales (Gelijns, 1998). Aspirin, for example, which has been used for over a century to relieve pain and fever, was more recently found to have important cardiovascular applications, deriving from its newly discovered roles in platelet aggregation and vascular inflammation.

Another common pattern is expansion of the relevant patient population for a given indication. Laparoscopic cholecystectomy, one of the most widely practised forms of

laparoscopic surgery in industrialised nations, is a case in point. A study, reporting on the experience of a large health maintenance organisation (HMO) in the Philadelphia area over a five-year period, found that 83% of patients with diseased gallbladders were opting for the laparoscopic procedure by 1992 (Legorreta, 1993). According to the HMO the cost of the operation had decreased by 25% over the period under review, but the HMO's total expenditures for gallbladder surgery rose by 18%. The reason was simple: associated with a 25% reduction in cost per patient was an increase in the number of gallbladder removals of no less than 60%. This much less invasive procedure enables physicians to remove the gallbladders of patients who, due to the frailties of age or the existence of co-morbidities, were previously regarded as too risky for the traditional "open incision" operation. Moreover, the laparoscopic procedure led to an increase in cholecystectomies for younger patients who were only mildly symptomatic.

The same pattern also appears in the treatment of clinical depression by selective seratonin reuptake inhibitors (SSRIs) - a major advance. Although not significantly more effective than their predecessor pharmaceuticals, SSRIs are considerably better tolerated. Patients are therefore more likely to continue therapy, which reduces the chance and duration of recurrence of disease. Along with this therapeutic advance came an expansion of providers, most of them outside the psychiatric field, who began to treat a heretofore untreated group of patients. This advance has expanded the treatment population, but the amount of overall morbidity reduction is estimated to outweigh the costs in a recent cost-benefit analysis (Frank, 1999). Generalisation is tricky however: expanding the use of an existing technology to a new target population may result in different costs and benefits and, thus, efficiency in the health-care budget could fall as well as rise.

Several themes emerge from this discussion. Firstly, new technologies when first introduced into practice are often relatively unfamiliar. Much improvement occurs as a result of downstream learning, which may lead to subsequent modifications in the technology itself or its application. Secondly, technological change can simultaneously reduce cost per patient, enhance quality and reduce risk to patients, thereby expanding the target population of use and/or demand. Thus, technological improvements that enhance efficiency are not necessarily accompanied by cost savings in health budgets or society.

Managing technology is not one but many tasks driven by multiple dynamics. The expansion of technological application in medicine is rarely homogenous and rates of adoption and use vary significantly between different regions and countries of similar economic development. What tools, then, are available to manage technological change?

Determinants of the uptake and diffusion of health-care technology

The rate of diffusion and the level of uptake of new health-care technologies are the aggregate outcomes of a large number of decisions made by politicians, health-care administrators, doctors and patients, just to mention a few. The political, organisational, and economic environments typically shape these decisions. That is, decisions that determine diffusion and uptake will be influenced by:

- *Aggregate income levels*: the amount of money available *i.e.* the overall level of income/size of GDP and the health-care budget.
- *Reimbursement mechanisms*: Financial incentives for purchasers to buy, and providers – both organisations and individual clinicians – to adopt, new technologies, including the way in which health care is financed and organised, *e.g.* competition between providers.

- *Regulation:* the regulatory environment.
- *Behavioural determinants*: the behavioural, organisational and cultural environments, *e.g*. the extent of local freedom to make decisions (as opposed to central control), the level of competition amongst health-care institutions, or a cultural imperative where "new is better" (versus conservatism in prescribing).

In addition, health technology assessment/evidence-based planning should be regarded as an overarching determinant on policy and practice because of its aim to influence decision making through the application of high quality evidence and appraisal of that evidence.

Some of these factors will vary in importance according to the technology being considered. For example, while drugs and devices are formally regulated to ensure their safety and functionality, the same is not true for all technologies. Moreover, drugs tend to be subject to greater post-regulatory scrutiny than other technologies, since they are regarded as one of the main drivers behind increases in health spending. The use of economic evaluation has often been targeted more at drugs than at other technologies.

Aggregate income levels

There is a considerable literature that examines the link between income (generally proxied by GDP per capita), health-care expenditure and the uptake and diffusion of health technologies. Health care is sometimes referred to by economists as a *luxury good*, meaning that societies might be willing to spend proportionally more on such goods as incomes go up.

A study by Slade and Anderson (2001), using data from 1975 to 1995, addressed the question whether richer countries are earlier adopters of new technology and whether differences in diffusion rates are maintained over time. The study examined the diffusion pattern of five new technologies in OECD countries and found a positive relationship between national income and the level and rate of technological diffusion. Higher income countries adopted new technologies earlier but, after the initial period of rapid diffusion, variations across countries declined. Possible explanations for this result include: *1)* in countries where incomes are lower, there is greater pressure on regulators to limit the rate of diffusion of expensive new medical technologies, and *2)* expensive medical technologies are more likely to be invented and produced in high income countries, implying an initial production location effect.

Lázaro and Fitch (1995) attempted to explain the differences in diffusion rates in terms of the aggregate measures of GDP and health-care expenditure per capita. Using linear regression, the authors found a significant correlation between GDP per capita and units per million populations (pmp) for computed tomography scanners (CTs), magnetic resonance imaging scanners (MRIs), linear accelerators (LAs) and radiation therapy units (RTUs). Health-care expenditure per capita was correlated with the level of diffusion for all these technologies, except CTs (although there was a strong correlation for this technology as well when the outlier, Japan, was excluded). However no correlation was found between health-care expenditure and the level of diffusion for extracorporeal shock wave lithotripters (ESWLs) and cobalt units (CUs).

The study also showed that there was no correlation between the technology diffusion and the number of physicians per capita. Significant variation in technology diffusion patterns remained unexplained even after differences in epidemiology were considered.

Whilst aggregate income and total health expenditure explains some aspects of technology diffusion, they remain beyond the control of most health-care policy makers. Even if total health expenditure could successfully be controlled, such a crude policy tool could not ensure that the most effective and efficient technologies are diffused.

Reimbursement mechanisms

The type of reimbursement mechanisms can have an important influence on the incentives for the uptake and diffusion of technology. Such incentives may encourage (or discourage) providers, patients and health-care institutions to use health-care technologies. Much of the evidence on the impact of different reimbursement mechanisms comes from the United States, since it provides an example of a natural experiment in contrasting methods of payment for health care.

An early study which examined the impact of prospective payment systems[2] on the decision by hospitals to adopt new "little-ticket" technologies is that by Romeo *et al.* (1984). It compared decisions made in three US states with, and three without, prospective payment systems. The authors examined adoption rates for five technologies and found that low prospective payment rates (in terms of hospital profits) had a negative effect on the adoption of the three cost-raising technologies. Low rates had a positive impact, however, on the probability of adopting one of the two technologies that would reduce costs. The study therefore shows that prospective reimbursement can speed or slow adoption, but that the impact varies according to the cost impact of the technology and the rate of prospective payment. Halm and Gelijns (1991) point out that under the US Medicare programme, coverage for a technology under its prospective payment system and the diffusion of the technology can depend crucially on the diagnostic-related group (DRG) to which the technology is assigned. Any difference between the reimbursement rate under the DRG and the cost of providing the technology will create an incentive in favour of, or disincentive against, providing the technology. The authors found that percutaneous transluminal coronary angioplasty (PTCA) was initially allocated to a surgical DRG, thereby providing a higher level of reimbursement than the cost of the procedure and stimulating its adoption. However, cochlear implants were reimbursed at a level lower than the cost of the device, leading to what they regard as "under-diffusion" and "adverse effects on subsequent R&D investment".

Similarly, in Japan, Ikegami (1988) has commented on the relatively high initial rate intended to promote renal dialysis; the rapid diffusion of this technology has been attributed primarily to the effect of a relatively generous rate of reimbursement.

Hirth *et al.* (2000) examined whether technological change can lead to savings through the reconfiguration of related services. The study looked at dialysis facilities which used quality-enhancing, but cost-increasing, technologies and considered whether the facilities had implemented cost-saving strategies that would have been feasible through the introduction of the new technologies. The cost-saving measures they observed were: *1)* an increase in the likelihood of facilities reusing dialyser membranes, *2)* a reduction in full time equivalent staff per patient and *3)* a shift towards lower-skilled labour.

2. Prospective payments set a fee per-case according to diagnosed medical conditions and standardised treatment costs. The best-known system is the diagnostic-related-groups (DRGs) where a patient is assigned to a group on entry to the hospital and the provider receives a lump sum for the treatment (Docteur and Oxley, 2003).

The cross-country analysis by Slade and Anderson (2001), outlined earlier, also considered the effect of the reimbursement system on the rate of technology diffusion. In comparing technology diffusion rates with the type of hospital reimbursement mechanism, they discovered that liver transplants, kidney transplants and haemodialysis were more likely to be provided where block grants rather than fee-for-service payment were used.

The type of reimbursement arrangements in place appears to have an influence on diffusion. Prospective payment systems can have a restraining influence on the use of technology but this is complicated by the observation that particular technologies can be encouraged (or discouraged) depending on the level of the prospective payment rate relative to the technology's cost.

Budgetary caps at the national, regional, or hospital level explicitly force decision makers to choose between technologies. In the United Kingdom, for instance, where decisions on capital spending occur at the regional level, National Health Service (NHS) administrators may have to decide whether to buy an MRI for one or two major centres or to purchase mammography equipment for, say, seven district hospitals. They may also have to make trade-offs between diagnostic and therapeutic technologies, which serve to dampen the overall rate of technology diffusion.

But budget-driven constraints on the general rate of diffusion of high-cost technologies do not necessarily trigger the selection of the most effective or cost-effective ones. A prerequisite for the rational allocation of resources within a fixed regime is information on the relative effectiveness and costs of medical interventions, and this detailed information is often not available (Williams, 1992).

In the past, medical and professional groups have often driven the system based on their understanding of the effectiveness of technology in delivering better outcomes, without explicit regard for cost. On the other hand, cost concerns have been left to managers in hospitals or other health authorities who, because they too have little information, appear to make arbitrary decisions in attempting to balance the fixed budgets set by public authorities. Recently, health-care systems have invested in strengthening the analytical enterprise to bring these two decision parameters (effectiveness and costs) together; for example, the United Kingdom created the National Institute for Clinical Excellence (NICE) that makes recommendations to the NHS about the use of particular treatments.

Regulation

It is widely recognised that different regulatory arrangements in different parts of the world can result in some countries having more rapid access to new drugs than others (PICTF, 2001). This section looks at how OECD countries use public planning and regulatory tools to provide, limit, or distribute the supply of medical technologies at the post-approval stage. For example, many OECD member countries require hospitals to obtain a public license for expensive devices and procedures. These planning mechanisms are directed chiefly at high-technology interventions, but are also used in the planning of facilities and manpower.

The implications of the state-based certificate of need (CON) legislation in the US for the diffusion of medical technologies have been well studied. CON laws have required hospitals to demonstrate need to state planning agencies for reimbursement approval. The effect of CON laws has been to allow individual state planning agencies to refuse

hospitals reimbursement for large items of capital expenditure unless the agencies consider there is a need for the service (OTA, 1986). Bryce and Cline (1998) report that CON requirements limited the supply of lithotripters to five units in Pennsylvania until 1986, when a change in legislation removed the restriction. Subsequently, the supply of machines more than doubled between 1988 and 1994, with an increase in procedures of 40%. Similar effects were found in the case of cardiac catheterisation laboratories and magnetic resonance imaging (MRI) machines.

A report by the Office of Technology Assessment (OTA, 1986) on the impact of CON legislation on the diffusion of extracorporeal shock wave lithotripters (ESWLs) argued that differences in the application of such regulations amongst states has mitigated the impact of such rules. Some states actively used their powers to withhold approval of some technologies whilst others were less restrictive. In some cases CON legislation was absent. It is argued that for the US overall, CON regulations have been insufficient to restrict diffusion to a level generally regarded as adequate. Possible reasons for this include widespread diffusion of the technology in health-care institutions outside the scope of the state-based CON legislation and the ability of patients to seek treatment across state borders.

Little evidence has been found on the impact of regulatory controls on technology diffusion outside the United States. However, Wild (2000) discusses the role of the Hospital Co-operation Fund (*Krankenanstalten-Zusammenarbeitsfonds*, KRAZAF) in Austria. Until 1996, this body was responsible for regulating the approval and purchase of expensive medical devices. The author notes that the use of large devices, particularly those relating to diagnostic imaging in hospitals, increased following the recommendations of two KRAZAF studies. However, the organisation was powerless to restrict the rapid diffusion of these technologies in the private sector, beyond the levels recommended by KRAZAF; in fact, insurers rewarded high-technology medicine, and in some instances large scale technologies were acquired for reasons of competition and prestige.

Planning tools can determine the geographic distribution of medical services, particularly technology with large capital requirements – sometimes referred to as "big-ticket" items. However, use of "small ticket" items (technologies with low capital cost requirements) is the major driver of the health-care budget, and it is not greatly affected by planning tools. Hence regulatory controls can often be seen as a potent but crude (necessary but not sufficient) tool for the rational allocation of technologies. Attention, therefore, turns to complementary methods for successfully integrating technologies.

Behavioural determinants

The work of Rogers (*e.g.* Rogers, 1995) focuses on the interaction between different individuals and organisations to explain the way in which innovations diffuse through social systems. Rogers argues that across a wide variety of areas of economic activity, diffusion typically exhibits an S-shaped pattern (Figure 2.1) whereby a few innovators adopt the innovation at first, followed by a group of early adopters who are opinion leaders in their field. Communication between these opinion leaders and their peers leads to take-off in the rate of adoption. It is argued that knowledge gained through this channel is a more important influence on the adoption decision than consideration of the scientific evidence. Differences in rates of diffusion can be explained partly by the perceived characteristics of the innovation. These include:

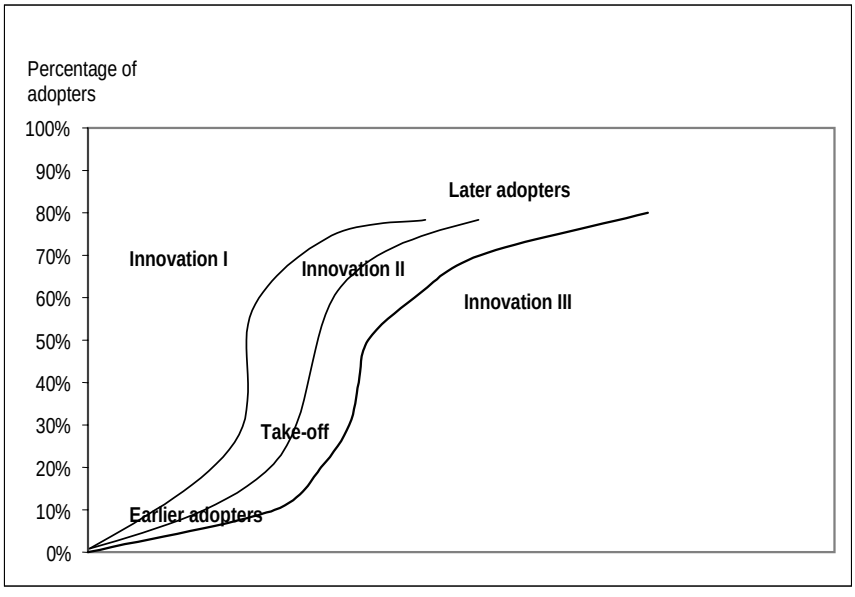
- Relative advantage: is it superior to existing practice?
- Compatibility: is it similar to previous experience or beliefs and values?
- Complexity: is it relatively difficult to understand?
- "Trial-ability: is it amenable to experimental use by an individual?

Greer (1988) found some support for the dynamic described by Rogers. In all the communities studied, Greer was able to identify innovators. These innovators frequently act as "idea champions" who actively promote the adoption of a new technology. At this stage, the role of the opinion leader who is trusted to evaluate the new technology is important in building a consensus around its adoption. It is suggested that these local actors are critical to the diffusion process and it is difficult for external sources of data in the form of, for example, the scientific literature, to change medical behaviour. In a number of countries, clinical opinion leaders are involved in the assessment and appraisal of health-related technologies, as well as the dissemination of HTA findings.

Ketley and Woods (1993) identified a clinician's involvement in trials of a technology as important in adoption decisions. For example, studies have shown that thrombolytic therapy reduces mortality after acute myocardial infarction (AMI). After the trial results were published, thrombolytic use was found to rise for several years from a low level, but a greater than two-fold difference remained between districts in rates of use per 1 000 patients admitted with AMI. This difference was strongly associated with districts' previous participation in multicentre trials of thrombolysis. In fact 64% of the variation between districts could be explained by their participation in trials in the previous two years.

A number of other studies have noted the S-shaped diffusion pattern, including work by Sillup (1992) and Hillman and Schwartz (1985).

Figure 2.1. **S-shaped diffusion curves**



Source: Rogers (1995).

Teplensky *et al.* (1995) investigated behavioural explanations for the rate of uptake of MRI at an individual organisation level. Using data from all US hospital-owned or sited MRI units in existence at the end of 1988, and a sample of non-adopter hospitals, the authors found the major influences on adoption behaviour to be the importance a hospital attached to being a technological leader, an emphasis on clinical services requiring MRI, and the change in revenues it believed was associated with MRI adoption. An aspect of provider behaviour not considered was the extent of interspecialty rivalry. Gelijns and Rosenberg (1994) consider this to have been an important influence on promoting technological change in particular conditions involving different medical specialties.

Organisational determinants

The literature has examined a number of influences on technology diffusion associated with the organisation of health care, such as the degree of competition among providers and hospitals. Some studies have also examined the impact of hospital size, location, ownership (public or private), and whether or not the hospital is affiliated with a medical school. In their study, Romeo *et al.* (1984) found that larger hospitals were more likely to adopt a technology. Teaching hospitals were also significantly more likely to adopt units, but no clear pattern emerged from public or private ownership, or the degree of competition among hospitals and providers.

In their study of dialysis facilities, Hirth *et al.* (2000) found that less competitive markets were associated with lower use of new technologies. These findings are echoed by those of Bryce and Cline (1998) in Pennsylvania. They argue that "hospitals have competed, in part, by acquiring technologies to attract and retain physicians and their patients" leading, for example, to "an oversupply of cardiac catheterisation labs".

Hill and Wolfe (1997) investigated the impact of managed care.[3] They found that two years after the start of the state of Wisconsin's HMO initiative, three hospitals in one area decided to share in the purchase of an MRI facility. Building a shared MRI facility reduced costs and stood a better chance of CON approval. The authors speculate that although managed competition may reduce expenditures on new capital equipment, the evidence is mixed. A higher percentage of people insured through an HMO appear to be associated with lower technology diffusion in ambulatory care facilities. However, it has little impact on technology diffusion rates in hospitals.

In the UK National Health Service, the "purchaser-provider split" reforms of the early 1990s meant that providers would compete for business from purchasers. The introduction of contracts between purchasers and providers, according to Rosen and Mays (1998a), presented the opportunity to use financial incentives to ensure that diffusion was evidence based. They argue, however, that most contracts have been "too crude to influence technological innovation".

The same authors (Rosen and Mays, 1998b) interviewed those who had been involved in the introduction of three case study technologies in three hospitals. These interviews indicated that in only one out of nine examples was there a purchaser-led introduction of new technology. The authors concluded that purchasers have a limited role in decision

3. Managed care can be defined as the body of clinical, financial and organisational activities designed to ensure the provision of appropriate health-care services in a cost-efficient manner. Managed care techniques are most often practised by organisations and professionals that assume risk for a defined population (*e.g.* health maintenance organisations) (Docteur and Oxley, 2003).

making when additional funding is unavailable - a situation which, they argue, is similar to that prevailing before the 1991 reforms.

Studies show that while the diffusion of some technologies follows an S-shaped curve as predicted by a particular mathematical model, this does not provide an explanation of differences in diffusion rates across time or geographical locations. More extensive diffusion tends to be associated with hospital size and teaching status. Again, however, the evidence on provider characteristics is not clear-cut. Similarly, whilst the purchaser-provider split creates greater incentives for efficient purchasing of technologies, the lack of more comprehensive information on which to base decisions and a lack of additional funding may impede such developments.

HTA and economic evaluation in decision making

The impact of HTA

One factor which cuts across the categorisation of policy tools presented here is HTA. As outlined in Chapter 1, HTA enables informed decision making in the diffusion and uptake of technologies. In principle, HTA activities, despite their relatively recent introduction into the policy-making environment, could have a significant part to play in the adoption and diffusion of medical technologies.

A concern of the evidence-based medicine community is the lack of correspondence between evidence and clinical practice (Haynes *et al.*, 1997). Various examples can be cited where technologies diffused rapidly before reliable evidence on effectiveness and cost-effectiveness was available. This was the case, for instance, with computed tomography (Drummond and Weatherly, 2000). A recent survey of HTA initiatives in the European Union concluded that "it is widely acknowledged that clinicians have not actually changed their practice to agree with HTA results" (Banta and Oortwijn, 2000).

Several interview-based studies have been undertaken to explore decision-making processes within individual health-care providers. Juzwishin *et al.* (1996) reviewed four such studies. They concluded that whilst a large number of respondents indicated that assessments were being conducted in their hospital, a significantly lower number used this information in subsequent acquisitions. This was partly due to the lack of organisational structures to implement HTA, and the absence of explicit criteria on which to base decision-making processes; such processes were described as "political", "informal" or "ad hoc".

However, other studies show that there are cases where HTA has had an impact. For example, Jacob and McGregor (1997) measured the impact that 16 health technology assessment reports had on policy decisions (*i.e.* the number of relevant regulations proposed, passed and enforced) and concluded that 12 of the reports had had considerable impact.

A review in Sweden found that there were changes in practice in line with HTA recommendations in six out of seven areas studied, though the authors (Britton and Jonsson, 2002) recognise that the level of impact of HTA depended on a range of circumstances, such as the level of acceptance of HTA recommendations by stakeholders, the level of reach of HTA reports, the timeliness of the report for decision making, the characteristics of the technology and the incentives within the health-care system that are aligned with the HTA recommendations.

In Quebec, Battista *et al.* (1999) documented the relationship between the Quebec Health Technology Assessment Council (CÉTS) and the decision-making process. They concluded that a CÉTS report on prostate cancer screening resulted in clinical guidelines being developed and "enabled the Ministry of Health and Social Services to decide not to launch a province wide screening programme".

In Alberta, Hailey *et al.* (2000) reported on the impact of a series of rapid HTA reports ("Technotes") prepared in response to specific requests from the provincial health ministry or health authorities. The policy issues which the reports addressed related to the possible referral of patients for treatment outside the province, the case for introducing new technology, the purchase of particular items of equipment and the appropriateness of existing clinical practice. Based on discussions with those making the requests and written feedback, the authors concluded that 14 out of 20 reports had exerted some influence on decision making.

These findings are similar to an earlier study by Hailey (1993) on the impact of detailed assessments conducted on 26 technologies by Australian advisory boards, mainly the National Health Technology Advisory Panel (NHTAP). Hailey found support for an influence on policy in 17 cases, and showed that there was an influence on practice for at least 11 technologies. For eight technologies, it was thought that the HTA had a major influence on policy and practice.

In the United Kingdom, the Department of Health has financed the production of a series of reports entitled "Effective Health Care Bulletins", which summarises evidence for decision makers in health care. In an evaluation of the impact of the bulletin on the treatment of persistent glue ear in children, Mason *et al.* (2001) estimated that 89 800 fewer procedures were conducted in the four years after the bulletin than would have been carried out otherwise.

In the last decade, cost-effectiveness has become part of policy makers' decision criteria in a number of countries. Typically, it is being used to determine whether a new drug is included on a publicly reimbursed formulary. Since the use of a new drug might be expected to depend heavily on whether or not patients have to pay for it themselves, this form of HTA should have a more readily observable impact on the diffusion of pharmaceuticals than, for example, clinical guidelines. It is certainly the case that claims for public reimbursement have been rejected. For example, Anis and Gagnon (2000) report that 65 of 88 submissions to the authorities in British Columbia between January 1996 and April 1999 were rejected.

However, as Menon (2001) points out, drugs not on the formulary in one province may be available in another and despite restrictions on reimbursement and other policy measures to contain costs, drug expenditures in Canada continue to rise. Similarly, in Australia, where a cost-effectiveness criterion was first introduced, Birkett *et al.* (2001) argue that drug costs continue to increase at a potentially "unsustainable rate". The overall effect on the diffusion of pharmaceutical technologies is therefore uncertain. Cost-effectiveness data can be used not only to restrict access to drugs or other technologies but as the basis for a positive recommendation of the technology, as has been illustrated by certain decisions made by the UK's National Institute for Clinical Excellence (NICE). In May 2004, NICE will publish an assessment of the impact of its guidance on the

utilisation, in England and Wales, of the health technologies which it reviewed between 2000 and 2003[4].

In summary then, there is some evidence on the use of HTA in individual health-care organisations but since formal assessment activities at a national or local level are a relatively recent development, there is little evidence of HTA's impact on clinical behaviour. Indeed, it is a frequent lament that HTA appears to have had relatively little impact on decision making. Nevertheless, some studies have found that formal HTA exercises can encourage changes in practice for particular technologies. This issue is gaining increasing recognition by decision makers and producers of health technology assessments. For example, the society of Health Technology Assessment International (HTAi) will devote considerable time to the issue of dissemination and the impact of HTA on policy and practice at its first conference in Krakow, Poland.

The use and limitations of economic evaluation

Over time, decisions have become more informed by evidence of effectiveness and cost-effectiveness. In Australia, for example, sponsors must provide cost-effectiveness data to obtain approval for public reimbursement of a new drug. In Canada, all publicly-funded drug benefit plans, with the exception of those in Quebec, participate in a common drug review (CDR) process. Under the CDR recommendations for common formulary listing are based on clinical and pharmacoeconomic reviews. The drug benefit plans make their own listing decisions based on these recommendations and other factors including the plan's mandate, priorities and resources.

Cost-effectiveness analysis (CEA) compares the costs and effectiveness of alternative treatments (*e.g.* technology A and technology B). The level of effectiveness can be measured using outcomes such as "life years saved" or "number of cases prevented". CEA can also incorporate a measure of morbidity in the level of effectiveness, using "quality adjusted life years" (QALYs) as an outcome measure.

The results of CEA are reported in a ratio. The numerator is given by the incremental effectiveness of technology A over technology B. The denominator is given by the incremental costs of technology A over technology B. The results of a CEA can be presented on the cost-effectiveness plane, shown in Figure 2.2. A CEA result in quadrant I should be interpreted as technology A having a greater level of effectiveness at a lower cost than technology B. In quadrant II, technology A has greater effectiveness but also greater costs. In quadrant III, technology A has greater costs but lower effectiveness, and in quadrant IV technology A has lower costs and lower effectiveness than technology B.

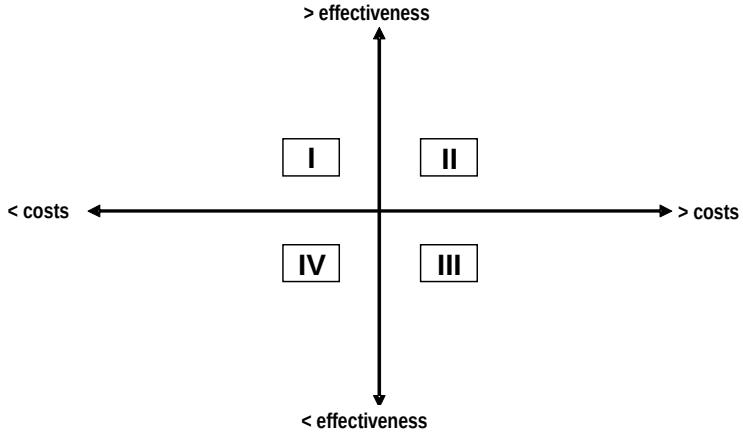
For the decision maker who is using economic evaluation to guide decisions, a CEA result in quadrant I is fairly straightforward; it would be desirable to implement a technology that is both more effective and less costly. Quadrant III is also fairly straightforward; it would be undesirable to implement a technology that is less effective and more costly. Quadrant IV would require a decision maker to sacrifice effectiveness for lower costs, and many decision makers would see this as undesirable.

A CEA result in quadrant II requires decision makers to decide how much more they are willing to spend for an additional level of effectiveness. It is easy to see why this would make decisions more politically complex. Decision makers have to make trans-

4. The report is available on the NICE Web site: *www.nice.org.uk*

parent choices about how much they value additional health benefits, which sometimes involves decisions of life or death.

Figure 2.2. **The cost-effectiveness plane**



One further potential application of cost-effectiveness analysis is in priority setting exercises. By first ranking all treatments by their CE ratio (see Table 2.1) and then starting with the one with the lowest cost per unit of outcome, payers can work their way down the list until their health budgets are exhausted. However, creating a social consensus about so-called league tables that include a wide range of interventions, such as childhood vaccines, artificial hips, and liver transplants, will be immensely difficult, and undertaking such an exercise may encourage political divisiveness by counter posing the interests of the young and the old, who have inherently less capacity (life expectancy) to benefit from interventions. Moreover, these and other groups are organised to assert their preferences and needs politically, and to protect their interests against efforts to brand them as lower priorities. Cost-effectiveness ratios can be a powerful aid to decision making, but because they do not capture such important factors as the impact on equity and distributive justice, these ratios cannot be used as an absolute cut-off criterion.

Table 2.1. Cost-effectiveness league table

Cholesterol testing and diet R_X	USD 330/QALY
Pacemaker implantation	USD 1 650/QALY
CABG (left main disease)	USD 3 135/QALY
Home hemodialysis	USD 25 890/QALY
Neurosurgery for malignant intracranial tumors	USD 161 170/QALY

QALY = quality adjusted life-year.

Source: Maynard, 1991.

Furthermore, even with greater emphasis on economic evaluations in decision making, considerable uncertainties over the cost and effectiveness parameters of treatments will persist, making decisions about coverage complex.

Some countries have introduced conditional coverage, whereby insurers pay the treatment costs of patients in an approved research protocol, while sponsors cover the costs of conducting and analysing the research. When the final research results are in, the insurer can make an informed, final decision about coverage. In the United States, two prominent early examples of conditional coverage include autologous bone marrow transplantation for end-stage breast cancer and lung volume reduction surgery for end-stage emphysema – both big-ticket surgical procedures.

Finally, cost and effectiveness parameters change over the life cycle of a technology, so decisions need to be revised regularly if they are to consider the latest and most accurate information. The significant fall in price of some stents in the use of percutaneous coronary interventions is a good example of this. Therefore, decision-making processes need to move away from the idea that a technology can be evaluated, once and for all, on the basis of research findings collected before its use in everyday practice.

Some concluding observations

This chapter has examined some of the mechanisms of innovation and the impact of innovation in the health-care sector. It has also reviewed the literature on the factors which affect the diffusion of health-care technologies. Policy makers have various instruments which can be used with some effect on the rate of technology diffusion. These can be planning tools and health technology assessment or incentive-based mechanisms, such as reimbursement arrangements. The current state of knowledge does not, however, provide a firm basis for estimating the impact of particular measures for a specific technology within the context of the overall health-care system. Nor will the rate of diffusion of technology in itself necessarily be of great interest to policy makers.

Whether the diffusion of a technology is deemed to be too fast or too slow, compared with its optimum rate, will depend on the decision maker's objectives, such as efficiency and equity. Where value for money is paramount, it will be important to ensure that cost-effective technologies become widely adopted and that those not found to be cost-effective do not become established. In this endeavour, HTA and economic evaluations such as cost-effectiveness analysis must have a role to play in identifying and disseminating information on best practice.

However, it would be a mistake to view cost-effectiveness and kindred analytical techniques as "answers" ready to be pulled off the shelf and implanted unproblematically into policy. Sources of cost data are less readily available than are measures of health outcomes. Current methods of resource-based costing leave much to be desired. Moreover, recent work on the statistical validity of cost-effectiveness ratios identifies deficiencies in that metric. Methodological alternatives (such as net health benefit) have only begun to be explored. Without epidemiological modelling that examines, for example, shifts from acute to chronic care and the implications of downstream learning for new populations and indications of use, the aggregate economic impact of technological change will remain hard to gauge with much precision.

Even if analytical methods were largely perfected, ethical and political challenges would give appropriate pause to policy makers seeking to implement them. One hears everywhere that such constructs as basic benefits packages reflect overdue recognition of the need for hard choices, made explicitly and publicly in the light of clear criteria and principles. One hears too that these hard choices should be participatory, inclusive,

consultative, in a word, democratic. But allowing these social virtues free play may make "hard" choosing impossible. What are the procedural requisites of an acceptable approach to explicit priority setting? Can democracy and technocracy co-exist peacefully?

A better understanding of the decision-making processes — how such processes can incorporate evidence as well as deal with issues of social justice and ethics — is important. Furthermore, more evidence on the partnership between decision making and HTA, as well as the effect that health-care policy tools have in facilitating (or impeding) the use of HTA, would be useful. These issues are explored further in subsequent chapters of this report.

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Chapter 3

THE PRODUCTION OF HEALTH TECHNOLOGY ASSESSMENT: AN ANALYSIS OF SURVEY RESULTS

The information produced by health technology assessment (HTA) can be used to enhance decisions about the use and diffusion of technology. This chapter presents results from the OECD survey on health technologies and provides information on how HTA is undertaken as well as its purpose, target audience and content. The methods used to disseminate the results of HTA are examined and a comparative picture is presented of national differences in the production of HTA.

Introduction

Advances in technology are responsible for great improvements in clinical, economic and social outcomes. At the same time, there are significant gaps in the evidence on the effectiveness and cost-effectiveness of technologies, many of which are of uncertain effect in terms of improving patient health (Fuchs, 1987; Maynard and McDaid, 2000). For example, a systematic review of 33 interventions to treat acute and chronic lower back and neck pain showed that in 44% of interventions there was no evidence, and in a further 29% there was only limited or moderate evidence of effectiveness. In 13% of interventions, there was evidence that the treatment was not effective (SBU, 2000). This lack of evidence creates difficulties for health-care decision makers when it comes to making informed choices about the uptake and diffusion of medical technologies.

The evidence-based medicine movement aims to create a greater evidence base and provide decision makers, especially clinicians, with the best available evidence on the effectiveness of interventions to prevent, treat and manage disease. The Cochrane Collaboration is probably the most famous example of such evidence-based medicine.

Health technology assessment (HTA) is a valuable source of information for a wide group of decision makers in health care, such as policy makers, health-care administrators and insurers. It is a form of assessment which not only considers the effectiveness of technologies but also aims to assess the wider implications, such as the social and legal consequences and the cost of technologies. Its primary function is to bridge the gap between scientific evidence and decision making (Battista and Hodge, 1999).

The information produced by HTA can be used to guide decisions about the use and diffusion of technology, thereby potentially improving efficiency and leading to better health outcomes. Furthermore, when HTA also considers the cost-effectiveness of technologies, it can guide[1] efficient resource allocation decisions. Without such evidence, the

1. Nevertheless, health technology assessment is not a substitute for decision making. Choices and trade-offs between objectives (*e.g.* equity and value for money) still have to be made. HTA can explicitly aid decision makers in making these choices.

uptake and diffusion of technologies are more likely to be influenced by a range of social, financial, professional and institutional factors, and may not produce optimum levels of health outcomes.

Many OECD countries are investing resources in HTA and evaluation activities. For example, the Dutch Fund for Investigative Medicine allocates EUR 8.6 million per year to health evaluations. Sweden spends EUR 5 million on its national agency, the Swedish Council on Technology Assessment in Health Care (SBU) and the French national agency for accreditation and evaluation (ANEAS) has an operating budget of nearly EUR 20 million (McDaid and Cookson, 2001). In Canada, combined spending on six HTA agencies totalled approximately CAD 12.35 million in 2000-2001 (Lehoux *et al.*, 2003), with an additional CAD 45 million committed over the next five years (2003-2008) to CCOHTA, the national HTA agency. The total budget for the Danish Centre for Health Technology assessment equates to DKK 49.8 million. In many instances, these programmes represent the substantial bulk of funding available to health services research and technology assessment. Yet these figures only represent between 0.01% and 0.04% of total national health-care spending (McDaid and Cookson, 2001; Lehoux *et al.*, 2003; OECD, 2003).

The small number of studies carried out show that HTA activities provide value for money. For example, Rosenau (2000) concluded that, overall, the Quebec Health Technology Assessment Council had an impact on clinical practice which saved around USD 25 million. In the United Kingdom, an evaluation of the "Effective Health Care Bulletin" which was dedicated to the treatment of persistent glue ear in children estimated savings of GBP 27 million (Mason *et al.*, 2001). Furthermore, the number of countries that have invested in and implemented HTA programmes provides some indication that HTA is widely valued. A recent formal evaluation of the Danish Centre for Evaluation and HTA reported that HTA is a cost-effective endeavour (National Board of Health, 2003).

With the additional investment comes a growing recognition that the practical use of HTA in policy and practice is paramount in determining whether HTA has been successful or not (Jonnson, 2002). More countries are placing greater emphasis on ensuring that the evidence from HTA (or other sources) is considered in the decision-making process. In addition, a number of countries have established national or regional institutes to co-ordinate and prioritise activities as well as improve dissemination of assessments.

This chapter looks at how HTA is *produced* in OECD countries. It presents the results from a survey[2] conducted as part of the OECD project on health-related technologies. The chapter begins by providing a background to HTA and examining why it has become such a widespread activity in a number of OECD countries. HTA considers the broad impacts of health technologies, including evaluations of benefits and costs (a fuller description of HTA is provided in Chapter 1). The survey provides information on how HTA is undertaken, in particular, the way it is conducted, its purpose, target audience and content. The methods used to disseminate the results of HTA are examined and a comparative picture is presented of national differences in the production of HTA,

2. Undertaken in Austria, Australia, France, Germany, Ireland, Japan, Mexico, the Netherlands, Norway, Spain and Switzerland. Telephone interviews were conducted in all survey countries, as well as Canada, to supplement survey responses.

focusing on two case study technologies: positron emission tomography (PET) and stroke technologies.

The growing need for HTA

A number of factors explain the demand for and supply of HTA activities:

- HTA arrived at a time when policy makers were becoming increasingly concerned about health expenditures, with technology perceived as a major driver of those costs.
- There was a growing concern over the possible ineffective (or even harmful) uses of untested technologies.
- The advent of randomised control trials and resultant availability of data led to strong methodological developments in HTA-related fields, including health economic evaluation techniques.
- The widespread variation in technology use has led to questions about the optimal use of technology – and as a consequence the need for evidence that allows decision makers to strive for optimum diffusion and uptake rates.
- The growth in medical research and technology, alongside developments in information technology, has made it impossible for decision makers (such as purchasers and medical practitioners) to keep up with all the new developments reported in the literature. There is a growing demand from decision makers for high-quality, comprehensive and manageable information, such as that provided by HTA.
- Trends in health system reforms have resulted in decentralised decision making, so that health-care choices are made closer to the patient. Against this background of devolving health-care decision making, the value of HTA is likely to increase. More decision makers throughout health-care systems will require access to high-quality evidence in order to make informed choices.

Overview of HTA production

Whilst there is considerable common ground on the broad definition of HTA, in practice HTA varies significantly amongst and within OECD countries in the way it is conducted, what issues are included within assessments and how it is used in decision making. To some extent this should not come as a surprise. If HTA is to bridge the gap between policy and evidence, then the way HTA is practised will reflect each health system's history, culture and values (Banta, 2003). It will have been designed and adapted to suit the specific needs of decision makers within a specific system. Hence, there are some significant areas of variation.

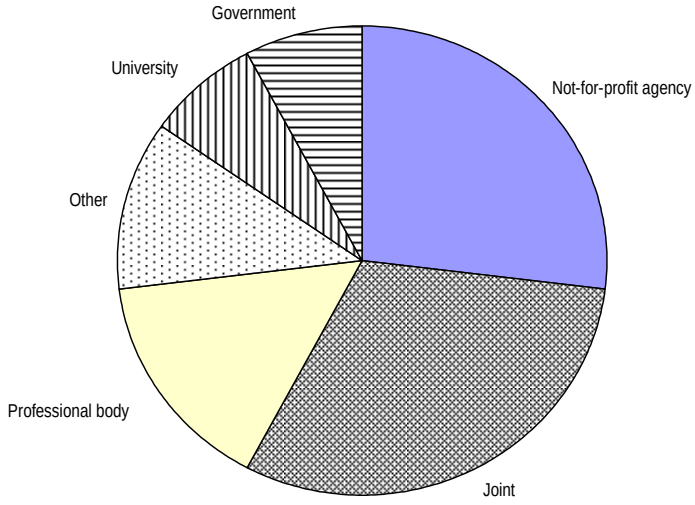
The following results are based on health technology assessments carried out on a sample of case study technologies. These technologies were selected on the basis that they would reflect the diversity of uses of health-care technologies (*e.g.* use of technology in screening, diagnosis, treatment, and management of disease) and health-care settings (*e.g.* ambulatory care, hospital care). The case study approach provides useful insights into the production of HTA in participating survey countries and enables comparative analysis of how survey countries have dealt with similar types of technologies. The

results shown in the remainder of this chapter are therefore representative of HTA production in survey countries, but do not purport to be a complete account.

Who undertakes HTA?

A number of different organisations carry out HTA. Figure 3.1 reveals that the most common practitioners were not-for-profit agencies, who conducted 27% of all HTAs in this sample (n=26). It also shows that joint assessments (more than one body involved) were common. Joint assessment always included a not-for-profit agency together with a professional body and/or government. The majority of the "other" categories in Figure 3.2 were hospitals who conducted their own HTAs.

Figure 3.1. **Type of organisations that undertook HTA for the case study technologies**



What is inside HTA?

Previous studies have concluded that many HTAs do not comply with the comprehensive definition of what constitutes HTA (see Chapter 1 for a description of HTA). The ASTEC[3] report, for example, concludes that while HTA has provided a vehicle for the delivery of information on the effectiveness of health technologies, information about cost-effectiveness has been more limited (Cookson *et al.*, 2000) A recent international comparison of HTAs characterised only 14% as a comprehensive assessment of the consequences of medical technologies (Poulsen, 1998). Such variation will, in part, reflect the information needs of decision makers and the objectives of the health-care system.

The OECD survey found that the content of HTA varied significantly for the five case study technologies. Table 3.1 demonstrates that while almost all HTAs included evidence of effectiveness (with only one exception in telemedicine), there were far fewer assessments that considered psychological, social and ethical factors. Cost-effectiveness was also reported widely, but additional costs or cost savings less so.

3. Analysis of the Scientific and Technical Evaluation of Health Care Interventions in the European Union.

Table 3.1. **Content of HTA by case study technology**

	PET	Hepatitis C genotyping	Telemedicine	Prostate cancer screening	Stroke technologies
Efficacy/effectiveness	100%	100%	83%	100%	100%
Quality/safety	83%	100%	83%	80%	100%
Psychological, social and ethical considerations	0%	33%	50%	20%	71%
Organisational and professional implications	33%	67%	100%	20%	86%
Cost-effectiveness	67%	67%	100%	60%	71%
Additional costs or savings	67%	67%	83%	40%	57%
Burden of disease in the population	67%	100%	33%	80%	71%
Severity of disease in the individual	33%	100%	17%	100%	86%
Equity	33%	100%	17%	40%	43%
Social benefits	33%	100%	50%	60%	71%
Patient perspectives	0%	0%	33%	40%	71%
Economic benefits	17%	67%	83%	40%	71%
Industry/R&D	0%	0%	50%	0%	29%
Waiting times	17%	33%	17%	20%	71%
Lack of alternative treatment	0%	0%	17%	20%	57%

There are many reasons why HTA content may vary, but it is difficult to determine which reason applies in each case. Sometimes there will be no actual data on which to report. For example, there are a number of reviews that emphasise the lack of effectiveness data for some telemedicine applications. Sometimes HTA will reflect the needs of different decision makers and the technology in question, and therefore place a stronger emphasis on some factors than on others. Cultural factors may also play a role here. For example, decision makers may not want costs reported explicitly, or they may wish to base decisions predominantly on effectiveness data. Finally, time and resources for HTA activities may also be determining factors in explaining variation of HTA contents.

What led to HTA being conducted?

The OECD survey asked respondents to identify the reasons for carrying out an HTA. Table 3.2 shows that in this survey the most common reason for undertaking an HTA was that it was part of an existing HTA programme. However, there is some interesting variation between technologies. For example, the primary reason for an assessment of costly technology such as Position Emission Tomography was a direct request from the government or insurer. However, an assessment of telemedicine was more likely to have

been conducted as part of an HTA programme. The other reasons for undertaking HTA included political pressure and priority for a national research or HTA programme.

Table 3.2. **Reasons for undertaking HTA by case study technology**

	Legislative/policy requirement	Part of an ongoing HTA programme	Voluntary/ ad hoc basis	Direct request from government/insurer	Other
PET	50%	50%	0%	83%	17%
Hepatitis C genotyping	67%	67%	0%	33%	33%
Telemedicine	0%	67%	33%	50%	17%
Prostate cancer screening	20%	60%	0%	40%	0%
Stroke technologies	33%	33%	17%	33%	50%
Overall	31%	54%	12%	50%	23%

Priority setting has become an important aspect of the HTA process. Countries set HTA priorities using a number of mechanisms. Under some programmes, such as the Australian Medicare and Pharmaceutical Benefit Scheme, priorities are set based on applications received from professional groups or industry.

In other countries such as Germany, Canada and the United Kingdom, processes have been put in place to receive suggestions for HTA topics from a wide range of stakeholders, including the public. In Canada, final decisions are made by a Board of Directors, representing the ministries of health across Canada, and based on agreed criteria. In Germany, a board of trustees, representing public administrators, patients and industry, comes to a resolution on HTA topics using a Delphi process. Under the UK's Health Technology Assessment Programme, advisory panels (consisting of members from a wide range of backgrounds, including consumers) recommend priorities to the Director of Research and Development.

Early identification programmes, often referred to as horizon scanning, may also be helpful in improving decision making with respect to technologies. Horizon scanning can provide important and timely information for decision makers by identifying new technologies and assessing the available evidence and gaps. Such programmes have been successfully launched in several countries, including Canada, the Netherlands, and the United Kingdom, as well as internationally through the EuroScan Network[4]. In the UK, the National Horizon Scanning Centre aims to provide advance notice to the Department of Health of new and emerging health technologies that might require urgent evaluation, consideration of clinical and cost impact, or modification of clinical guidance activities.

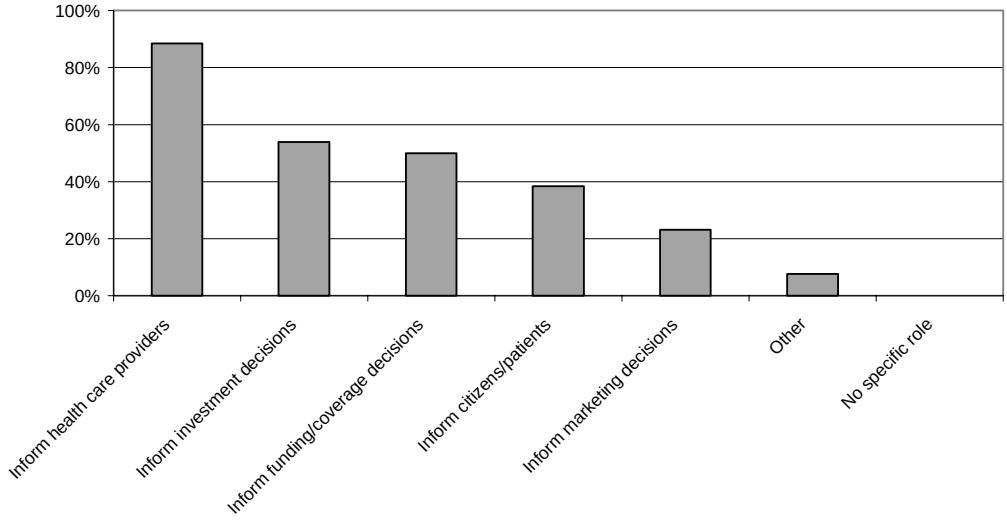
4. The Euroscan network comprises twelve members, primarily technology assessment agencies, in ten countries, including two outside of Europe (Canada and Israel). It aims to establish a network to: evaluate and exchange information on new and changing technologies, develop the sources of information used to identify new and emerging technologies, develop applied methods for early assessment, and disseminate information on early identification and assessment activities.

Who and what is HTA for?

The role of HTA varies by country. Some countries have instigated (or are in the process of instigating) formal consideration of economic evidence in decision making, particularly for pharmaceutical reimbursement decisions. For example, economic evaluation in support of listings under publicly-funded benefits began in Australia in 1993 and have now been introduced in one form or another the following OECD member countries: Australia, Belgium, Canada, France, Italy, Japan, the Netherlands, Portugal, Sweden, Switzerland and the United Kingdom. (Dickson, Hurst and Jacobzone, 2003).

Beyond pharmaceutical technologies, Figure 3.2 shows that the most commonly envisioned role for HTA was to provide information to providers. This was closely followed by HTA's role in informing decisions regarding investment/acquisition of health technology.

Figure 3.2. **The purpose of HTA**



The timing and resources required for HTA

The survey asked respondents to estimate the time it took to complete the HTA. The results from 19 different HTAs show that some HTAs took less than a month while others took up to four years. Table 3.3 reports the percentage of HTAs in the survey by the amount of time taken.

Table 3.3. **Time to undertake HTA**

Time taken	Percentage of HTAs (n=20)
Less than a month	5%
Between 1 and 3 months	10%
Between 3 and 6 months	30%
Between 6 and 12 months	20%
Between 1 and 2 years	20%
Between 2 and 4 years	15%

The variation in the length of time taken to conduct HTA can be explained by a number of factors. For instance, some respondents reported that some HTAs were "overviews of overviews", delivering rapid and inexpensive assessments. The lack of evidence around key decision parameters meant that in some countries, resources were made available to conduct primary research and trials. Such studies took longer, and were considered to be part of the HTA activities reported in Table 3.4. In other instances, HTA consisted of a literature review conducted by a single researcher. The estimated resources used to undertake the HTA ranged from as little as EUR 12 000 to EUR 2 million for a multiphase international trial. The available resources and time for HTA may impact on the quality and comprehensiveness of the HTA reports, though it is not possible to make any firm conclusions about this based on survey information or the existing literature.

A number of survey respondents noted that one of the biggest challenges for HTA was the speed with which technologies develop and can make HTA results obsolete. For some technologies, the dynamic pace with which technologies change and develop necessitates the development of HTA methodologies which are suited to such change.

These comments could point towards the need for guidance on how assessment can be undertaken for technologies that are at different stages of development. Such guidance may involve an examination of appropriate ways that HTA is conducted and reported, based on the maturity and characteristics of the technology.

Distributing HTA activities

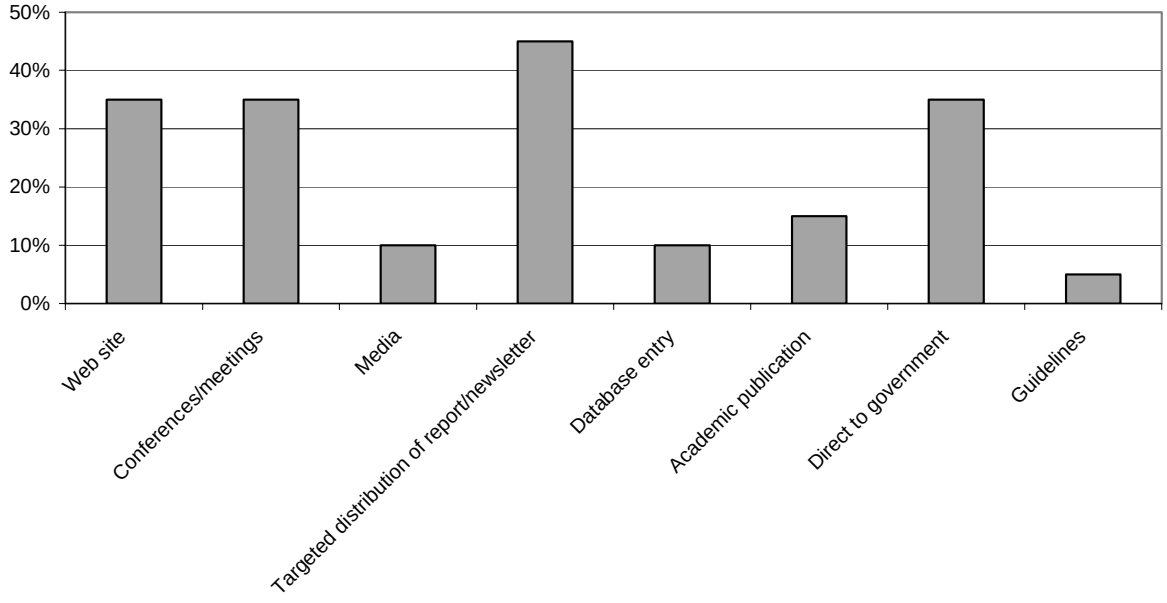
Decisions about health technologies are made by a range of actors. Whilst some OECD countries use centralised decision making, this is usually restricted to coverage and reimbursement-type decisions. However, OECD health systems empower clinicians to make decisions with their patients, within the parameters set by the overall health system structure.

Given that the ultimate decision whether or not to employ a given technology is devolved, HTA practitioners recognise that part of their role is to ensure that the evidence can be used by such local decision makers.

Figure 3.3 shows that most HTA practitioners use multiple means of distributing the results of HTA (n=16). The most common forms of dissemination were: widely distributing reports or newsletters, Web site publication, and conferences and meetings. A number of respondents mentioned that they used different methods of dissemination to

target various audiences. That is, they established a portfolio approach to dissemination depending on the key messages of the HTA and who the decision makers were.

Figure 3.3. **Methods of disseminating HTA**



More generally, a number of HTA agencies have introduced programmes aimed at improving the *receptor capacity* of HTA. The Canadian Coordinating Office of HTA, for example, has identified the development of receptor capacity as a strategic priority. This essentially means developing the demand for HTA by decision makers. Developing receptor capacity involves educational programmes directed at policy makers, educational institutions, medical practitioners and perhaps the public (Sanders, 2002).

Similarly, the Swedish Council of Health Technology Assessment (SBU) has appointed HTA ambassadors in Swedish regions to carry out intensive dissemination activities, including, for example, visits to physicians to discuss HTA reports.

There is little doubt that successful implementation of HTA and evidence-based medicine requires a cultural shift towards the use of evidence in policy and practice. This is supported by a recent Canadian study that found that personal contact with an HTA agency was one of the most important factors in raising awareness of HTA activities. The study also found some evidence that a portfolio approach to dissemination may be more effective than a single dissemination strategy (Lehoux *et al.*, 2003). Although we still do not know which dissemination methods work best. This perhaps calls for a need for greater investment in evaluating the effectiveness of various HTA dissemination practices.

The production of HTA in OECD countries: a review of two case study technologies

The remainder of this chapter looks at the production of evidence in OECD countries for two case study technologies: PET and stroke technologies. It briefly describes each technology, examines the number of publications about the two technologies (including those with economic content)[5] and provides a summary of the main conclusions found in HTA reports[6]. Finally, this section of the chapter presents comparative survey results on the production and dissemination of HTA for the two technologies.

Positron emission tomography (PET)

PET is a minimally invasive nuclear medicine technology that enables the visualisation and measurement of biochemical processes within tissues. Like other nuclear medicine techniques, PET makes it possible to measure local tissue and organ function. It can complement the information obtained from other imaging methods, such as computed tomography (CT). These two techniques have recently been combined as PET-CT.

PET uses a radioactive tracer to assess perfusion and metabolic activity within the human body. It requires a charged particle accelerator, usually a cyclotron, to produce the radioactive tracers. PET is considered a large capital expenditure.

PET was first recognised as a valuable basic research tool and subsequently as a clinical tool. Initial use of PET was in neurology, then cardiology, but the greatest use is currently in oncology.

The production of evidence for PET

The initial article about PET was published in 1969, and the first article with economic content was published ten years later in 1979. The number of articles on PET increased rapidly from 1979 onwards. In 1980, 166 articles were published, increasing to approximately 1800 per year in 2002. Overall, less than 2% of all published PET articles included economic information. A slightly higher percentage of economic evidence can be found in more recent publications.

Thirty-three PET HTA reports were found on the INAHTA database[7]. The first HTA report was published in 1994 but most have been published since 2000. The majority of HTAs focus on the use of PET in the diagnosis and treatment of cancer, but some address indications for neurological disease and cardiac viability.

The early HTAs suggested that the evidence for the use of PET was limited. The technology was typically described as "in development" and promising, and was recommended for limited use within the framework of research and clinical trials. Some HTAs recommended that funding for PET be tied to further research and data collection.

5. Medline was used to count the number of citations.

6. The INAHTA database was used for this search. Database available at *www.york.ac.uk/inst/crd/htahp.htm.*

7. International Network of Agencies for Health Technology Assessment. The database can be accessed via the INAHTA Web site: *www.inahta.org*.

In 1999, INAHTA reviewed the use of PET in the fields of neuropsychiatry, Alzheimer's disease, brain tumours and cardiology (Adams *et al.*, 1999). Whilst the review found that PET could be superior to other imaging techniques in these fields, improved clinical outcomes had not been demonstrated. However, the INAHTA review suggested that PET may be useful in planning treatment in oncology, even though there were conflicting results for its use in the staging of non-small cell lung cancer: some agencies had concluded that the use of PET was potentially cost-effective while others found the evidence was not sufficient to establish efficacy.

Several other HTAs, conducted around the same time, suggested that the most important use of PET may be in oncology. Since then further evidence has suggested that PET is useful in the diagnosis and staging of cancer, and modelling has also shown that it may prove cost-effective in the diagnosis and staging of lung cancer. The indications for the use of PET are broadening and a large number of studies are underway.

The use of PET is increasing with some HTAs describing it as "an essential technology in cancer". Other studies note that although the evidence is limited, the technology has several uses which other technologies cannot reproduce. The available, albeit limited, economic evidence suggests that while PET might not be cost saving, it may be cost-effective (MSAC 2001, AETMIS, 2001) (*e.g.* quadrant II as described in Figure 2.2 in Chapter 2).

Of the 12 survey countries, eight have conducted an HTA on PET. Table 3.4 reveals when the HTA was conducted and, where data was available, how long it took and when the technology was first used in each country. In most cases the first use of the technology was closely followed by the publication of an HTA report. Only in Germany and Canada was there a significant gap between the first use of the technology and an HTA, perhaps reflecting the early establishment of that technology and the relative lack of HTA activity at that time. Table 3.4 also shows that five of the eight survey countries conducted multiple HTAs on PET. Such repeat HTAs provide an economical means of delivering up-to-date information on the latest evidence and technological developments.

Other survey countries have also been active in the production of evidence for PET. In the Netherlands, Canada and Switzerland, projects are underway to study its effectiveness and cost-effectiveness. In addition, HTAs on PET have been conducted in the United States, United Kingdom and Denmark.

Table 3.4. **PET HTA production in survey countries**

	When was technology first used?	When was HTA reported/published?	How long did it take?
Norway	1998 (approx.)	1999, 2003	1999: 6 months; 2003: 2 months
Ireland	2002		1 month
Germany	Early 1980s	2002	4 years
Spain	1995	1998, 1999, 2001	2001: 6 months
Japan	After 1997	2002	
Australia	1995	2000, 2001, 2002	6 months
France	1999	1998, 2001, 2002	
Canada	1990	1998, 1999 (Alberta); 2002 (Quebec)	

Table 3.5. **Content of PET HTA in survey countries**

	Norway	Ireland	Germany	Spain	Japan	Australia	France	Canada (Quebec)
Efficacy/effectiveness	√	√	√	√	√	√	√	√
Quality/safety	√	√		√	√	√	√	√
Psychological, social and ethical implications								
Organisational and professional implications	√	√					√	√
Cost-effectiveness	√	√	√			√		√
Total cost burden		√	√	√		√	√	
Burden of disease			√	√	√	√		√
Severity of disease					√	√		
Equity considerations		√			√			
Social benefits				√	√			
Patient preferences								
R&D/industry								
Waiting times				√				√
Lack of alternative treatment								

Table 3.5 shows the topics covered in the PET HTAs conducted in survey countries. All HTAs covered the topic of efficacy/effectiveness and five covered cost-effectiveness. No HTA covered patient preferences, yet it could be envisaged that these may prove important in the assessment of PET given that the technology is non-invasive and a potential substitute for surgery. Similarly, PET has important organisational implications, including workforce training, access to a cyclotron to produce isotopes, and agreements between facilities on the shared use of the technology. A number of HTAs[8] recognised this and made planning and organisational recommendations for the deployment of PET.

Significantly, most HTAs commented on the lack of persuasive data on the technology's efficacy and cost-effectiveness and recommended its careful deployment, preferably tied to further research into its effectiveness and cost-effectiveness.

8. For example, those conducted by *l'Agence d'Evaluation des Technologies et des Modes d'Intervention en Santé* (AETMIS, Quebec) and *le Comité d'Evaluation de Diffusion des Innovations Technologiques* (CEDIT, France).

PET can still be considered a technology in development. There are expanding indications for the technology and developers have recently fused PET with CT scanners for more complete diagnostic information. Reflecting the expanding evidence base, the growing number of PET uses and indications, and technological development, a number of countries (see Table 3.4) have conducted multiple HTAs. HTA can potentially be used to communicate important information to the innovation community. HTA could highlight areas of uncertainty and suggest areas for further research. It could also outline aspects of the technology where changes might be needed to improve, for instance, its cost-effectiveness: quicker scans could lead to higher throughput and result in improvements in the cost-effectiveness of the technology.

Table 3.6 shows the envisioned role for, and the target audience of, PET HTA in survey countries. Most countries conducted PET HTA for the purpose of reimbursement and coverage decisions. The role of HTA was to also provide information to health-care providers, even though this group was not always the primary target audience. This may indicate that informing providers is a secondary goal for some countries.

Table 3.6. **What and who is the PET HTA for?**

	Norway	Ireland	Germany	Spain	Japan	Australia	France
What was the envisioned role of the HTA?							
Inform reimbursement/ coverage decisions	√	√	√	√	√	√	
Inform health-care planning/ investment decisions		√					√
Inform patients or citizens							
Inform providers	√	√		√	√	√	
Who represents the target audience of the HTA?							
Political decision makers	√	√	√	√	√	√	√
Third party payers					√		
Hospital managers/ administrators	√	√		√			√
Health-care providers				√	√		
Patients/citizens							

The most common target audience for PET HTAs comprised political decision makers and hospital and health-care administrators. This is perhaps not surprising, given the capital and recurrent costs required for the installation and operation of a PET centre[9]. Funding and investment decisions regarding PET may very well go beyond the means of a single hospital and need to be made at a higher level in the health-care system such as the provincial or national departments of health.

9. For example, CEDIT reports that capital expenditure for a PET system amounts to EUR 1.9 million plus further costs for layout work. The yearly operating budget is in the region of EUR 300 000.

Table 3.7 demonstrates the methods used to disseminate HTAs reports. Most HTAs were disseminated using a variety of techniques: extensive use was made of the World Wide Web, newsletters and e-mail alerts, as well as databases such as those established by INAHTA. Four of the HTAs had a direct link to decision making (either funding or planning/investment decisions).

Table 3.7. **PET HTA dissemination techniques**

	Norway	Ireland	Germany	Spain	Japan	Australia	France
Web site	√		√	√		√	√
Conferences							
Media							
Targeted distribution of report/newsletter	√			√	√	√	
Database entry	√		√	√		√	√
Academic publication			√				
Direct link to decision maker		√	√			√	√
Guidelines							

Stroke technologies[10]

Stroke is a leading cause of morbidity and mortality within developed nations. It can be caused by decreased blood flow to a portion of the brain (ischemic) or by haemorrhage within the brain (haemorrhagic). Both causes produce infarction or death to a portion of the brain. The majority of strokes are ischemic in nature (85%). The clinical signs of stroke depend on the area of the brain affected but include numbness, weakness, speech and vision disturbances. The incidence increases with age; risk factors include hypertension and diabetes. Strokes consume significant resources in treatment and long term care, although with the improving management of risk factors, this consumption is decreasing. Recently, thrombolytic therapy has been considered for treatment within three hours of the onset of acute ischemic stroke.

The care for patients suffering from stroke involves acute care in the initial stages and rehabilitative care over a longer time period. A variety of different organisational models can support this. One possible type of specialist organisation model is the stroke unit. This is where a multidisciplinary group of specialist staff look after stroke patients exclusively, rather than caring for them alongside other patients, either in general or geriatric wards. A stroke unit may cover the acute phase, the rehabilitative phase or both. Careful discharge planning is also a feature of stroke units. Stroke services, such as those offered in the Netherlands, go beyond the hospital phase of care and include broader community services that are delivered post hospital discharge.

10. Most survey countries focused their attention on stroke units for this case study. The Netherlands took a broader approach and highlighted their experience in stroke services, while Norway focused on the use of thrombolysis in the treatment of stroke.

The production of evidence for stroke units

Stroke units were first cited in 1969 and the first citation containing economic information appeared in 1979. Relatively few articles were published before 1992 and the growth in literature has been relatively slow. In 2002, there were 67 citations for stroke units and, of those, only three included economic references.

Six HTAs on the topic of stroke units were identified in the INAHTA database and published between 1992 and 2003. The first of these reports suggested that while stroke units improved patient outcomes, further evidence was required. They used the term "stroke unit" to deal with a wide variety of different types of service arrangements, including services that go beyond hospital care (*e.g.* community services). The early HTAs which reported on trials of stroke units that included intensive care services were disappointing. Results for rehabilitative stroke units, however, were more encouraging, even though it was not obvious which component of stroke unit services was driving the results.

Later HTAs carried out between 1999 and 2003 concluded that stroke units improve mortality and that the evidence for this is convincing. Patients who attended rehabilitative stroke units were more likely to be alive and living at home than patients who received services on the general ward. An influential Cochrane meta-analysis supported this finding. Furthermore, these later HTAs reported that there was some evidence that the cost of care in a stroke unit was comparable to that in a general medical ward (*e.g.* quadrant I or II in Figure 2.2) (Noorani *et al.*, 2003).

The Norwegian focus of stroke technologies was on using thrombolytic therapy in the treatment of stroke. An HTA report produced by the Norwegian Centre for Health Technology Assessment (SMM) concluded that the evidence for thrombolytic therapy in the treatment of stroke was uncertain and there was a risk of intercranial haemorrhaging as well as inappropriate administration of the medication. In appraising the evidence, the Norwegian working group also found that the treatment was expensive and represented a significant opportunity cost. They concluded that such treatment of stroke patients should be carried out in the form of new randomised controlled clinical studies (SMM, 1999).

Table 3.8 indicates when survey countries produced HTA reports on stroke technologies. Note that the Norwegian HTA refers to thrombolytic therapy and the Dutch HTA to broader stroke services, beyond those offered inside hospitals. The Dutch HTA also included primary research following a pilot programme offering different models of stroke care across the country. The evaluation of these models focused on developing the optimum model of care for national implementation and included cost-effectiveness analysis.

There was a significant gap between the time when stroke units were first conceived and their introduction into the health-care system. HTA reports were published fairly close to the date of the initial introduction of stroke units. Table 3.8 also demonstrates that no survey country has conducted repeat HTAs for stroke units.

A further complicating factor in the assessment of stroke units is that the range of interventions differs over time and also within and amongst countries. It is therefore difficult to know which aspects of stroke unit services are effective in delivering better health comes and which are less important. This complicates comparative analysis of effectiveness and cost-effectiveness and impedes the generalisability of study results

between settings. Further research in designing an optimum package of services contained within stroke units is required.

Table 3.8. **Stroke technologies HTA production in survey countries**

	When was the technology first used?	When was an HTA reported/published?	How long did it take?
Norway	1994 (in trials)	1999	Approx. 4 months
France	1985	2000	18 months
Netherlands	2002		3 years
Mexico	1997		6 months
Germany	1994	2000	6 months
Canada		2003	

Table 3.9. **Content of stroke technologies HTAs**

	Norway	France	Netherlands	Mexico	Germany	Canada
Efficacy/effectiveness	√	√	√	√	√	√
Quality/safety	√	√	√	√	√	√
Psychological, social and ethical implications	√		√	√		
Organisational and professional implications	√	√	√	√		√
Cost-effectiveness			√	√	√	√
Total cost burden				√	√	
Burden of disease	√	√		√		
Severity of disease	√	√	√	√		
Equity considerations		√		√		
Social benefits		√	√	√		
Patient preferences			√	√	√	√
Economic benefits		√		√	√	
R&D/industry						
Waiting times		√	√	√		
Lack of alternative treatment		√		√		

Table 3.9 reveals the topics covered in HTAs on stroke technologies in survey countries. All HTAs covered the topic of efficacy/effectiveness and four covered cost-effectiveness. Compared to PET HTAs, stroke HTAs covered a wider set of topics including patient preferences and social implications. This may reflect the wider impacts that stroke has on the carers of patients and the availability of well developed tools to measure patient functioning following stroke. Similarly, most stroke HTAs covered organisational implications, perhaps in recognition of the complexities of integrating multiple disciplines and services into a cohesive unit.

Despite the long timeframe over which stroke units have been developed, a number of HTAs stated that there was still considerable uncertainty about its cost-effectiveness.

Table 3.10. **What and who is the stroke technologies HTA for?**

	Norway	France	Netherlands	Mexico	Germany
What was the envisioned role of the HTA?					
Inform reimbursement/coverage decisions			√	√	√
Inform health-care planning/investment decisions		√	√	√	√
Inform patients or citizens		√	√	√	√
Inform providers	√	√	√	√	√
Who represents the target audience of the HTA?					
Political decision makers	√		√	√	√
Third party payers			√		√
Hospital managers/administrators		√	√	√	√
Health-care providers	√		√		√
Patients/citizens			√	√	

Table 3.11. **Stroke technologies HTA dissemination techniques**

	Norway	France	Netherlands	Mexico	Germany
Web site	√	√	√		√
Conferences			√		√
Media	√				√
Targeted distribution of report/newsletter	√	√	√	√	√
Database entry	√	√			
Academic publication			√	√	
Direct link to decision maker		√	√		
Guidelines					

Table 3.10 provides details on the envisioned role for, and the target audience, of HTAs relating to stroke technology in survey countries. Most countries conducted stroke unit HTA for the purpose of planning and investment decisions, as well as to inform health-care providers.

The most common target audience for stroke technologies HTA comprised political decision makers and hospital and health-care administrators. This could indicate that the establishment of stroke units requires decision making at the hospital level and also political support to bring together various institutions and disciplines. The Norwegian HTA, which recommended against the use of thrombolytic therapy for stroke patients, was directed primarily at informing providers.

Table 3.11 reveals the methods used in survey countries to disseminate the results of the stroke HTA report. The most common form of dissemination was targeted distribution through e-mail alerts, newsletters and publication on a Web site. Two countries reported a direct link to the decision maker.

Lessons from PET and stroke technologies HTA comparisons

There are many similarities, but also some important differences, in the production of evidence on PET and stroke technologies.

For both technologies, there was a similar time gap between the initial "development" of the technology and its first use in survey countries. Furthermore, both technologies reportedly have significant gaps in the available evidence, including the economic evidence. Nevertheless, a number of HTAs have tentatively concluded that PET may represent value for money, and a recent HTA on stroke units indicated that these may take up a similar amount of resources as the alternative general care.

Table 3.12 indicates that the first citing of evidence occurred around the same time for both technologies, as did the first publication of economic evidence. Yet despite the similar "age" of the technologies, the number of publications differs markedly. In 2002, for example, there were 1800 citations for PET but only 67 stroke unit publications. In total, there are forty times as many PET citations as stroke unit citations. The INAHTA database shows that the number of PET HTA citations still outnumber stroke unit HTAs, but the difference was significantly smaller (33 versus 6).

Several reasons might explain this, including a study bias towards technologies that are marketable and reimbursement policies that create incentives to study marketable technologies.

Table 3.12. **Number of citations and HTA reports for stroke units and PET**

	Total number of citations 1966-2002	Total number of economic citations 1966-2002	First citation	First economic citation	Number of HTA citations
Positron emission scanning	19 708	455	1969	1979	33
Stroke units	490	35	1969	1979	6

The content of the HTAs varied significantly depending on the technology. Whilst all HTAs considered issues of effectiveness and efficacy, the stroke unit HTAs generally covered a wider set of topics, including patient preferences. This could reflect the availability of measurement tools to assess human functioning following stroke care. However, the use of PET technologies can also have important implications for patient preferences, aside from health outcomes. For example, patients may prefer the non-invasive characteristics of PET, rather than exploratory surgery.

Finally, four survey countries conducted HTAs on PET on more than one occasion, whereas no country examined stroke units more than once (despite the considerable uncertainty in the early days of their development). Several key producers of HTA have commented on the lack of evaluation resources to re-examine technologies even though the value of doing so was considered to be high, especially for a technology which was in development.

Discussion

Health technologies can convey enormous benefits to health, but may account for up to half of the increased costs in health care. Health-care decision makers face the challenging task of harnessing the opportunities created by health technologies and at the same time ensuring that the health-care system remains sustainable and equitable. Adding to this challenge are the sometimes conflicting pressures and demands from patients (and tax payers), health professionals, the producers of new technologies, and a range of other pressure groups.

Objective evidence is of crucial importance in reaching transparent decisions and help decision makers resolve some of the many conflicting demands on health-care policy. Without evidence, the uptake and diffusion of technologies are more likely to be influenced by a whole range of social, financial, professional and institutional factors, and may not produce optimum levels of health outcomes. Recent efforts such as the Cochrane Collaboration and health technology assessment are viewed as valuable sources of information for decision makers.

The focus of this chapter has been on the production of health technology assessment. HTA warrants this focus because it aims to provide more comprehensive information to decision makers. HTA is a form of assessment which considers not only the effectiveness of technologies, but also their wider impacts, including legal and social issues and efficiency.

The survey results presented in this chapter point to key "success factors" that improve the extent to which HTA is likely to be taken account of in decision making. These success factors do not suggest a single homogenous model of conducting and implementing HTA. Instead, HTA needs to be adapted to national or local contexts. Nevertheless, the success factors outlined below reveal that important lessons can be drawn from international experiences and may be exported from one setting to another.

Investment in clinical evaluative research and HTA

Despite its growing importance, clinical evaluative research and HTA account for very small proportions of total health-care spending. Several studies highlighted that HTA has been a "value for money" activity. However, developing a culture of evidence-based medicine and policy requires secure and long-term investment to ensure the appropriate training of the workforce, the development of expertise, and the development

of methodologies that build on the quality and relevance of HTA to decision makers. Such investment should recognise the need for better guidance on how assessment, including repeat assessment, can be undertaken for technologies that are at different stages of development. This may involve examining appropriate ways that HTA can be conducted and reported, depending on the maturity and characteristics of the technology.

Dissemination activities

Significant proportions of HTA activities need to be devoted to the dissemination of results. HTA reports are disseminated through a variety of means including the internet, e-mail alerts, conferences, newsletters, education campaigns, media and personal contacts. There is only limited evidence on the dissemination activities that are most effective at reaching decision makers. However, there is some evidence which suggests that a portfolio approach to dissemination may be the most effective means of reaching numerous health-care decision makers.

Successful dissemination may also depend on establishing a culture of evidence-based policy and practice. Activities here may involve what has become known as building *receptor capacity*. Education programmes can be targeted at developing decision makers' skills in interpreting and analysing evidence, and establishing information infrastructure to make evidence more readily available. A further avenue to ensure greater use of HTA is to develop HTA programmes with a more defined function in the decision-making process.

Measuring the use of HTA

There is a need to develop more cohesive frameworks for analysing the extent to which HTA has helped decision makers make rational choices. An international framework of analysis, with agreed performance indicators, would create greater opportunities to develop best practices in encouraging the use of HTA in decision making. The development of such a framework could help deliver the efficient production of HTA and ensure that its use in decision making is optimised. .

Delivering decision makers' information needs

In order for HTA to influence decision makers, it has to produce the evidence that they require. This means ensuring the timely availability of information, in line with decision priorities, and recognising the various dynamics of different technology markets. The involvement of decision makers early on in the assessment process may help deliver more valued and relevant information. However, there may be occasional tensions between HTA methodology and decision making. On the one hand, there is support for autonomous and rigorous HTA with sound methodologies. On the other hand, there is a recognised need to ensure that HTA is suited to the decision-making process and the decision makers' needs – which sometimes (although certainly not always) may be at odds. Timeliness of HTA production is good example where sometimes HTA producers and decision makers' needs diverge. This point underlines the need for better communication between the producers and users of HTA to ensure sound methodology and relevance.

Furthermore, HTA can potentially be used to build better links between decision makers and the innovation community. For example, HTA highlights areas of uncertainty, suggests areas for further research, and outlines aspects of the technology where changes might be needed to improve, for example, its cost-effectiveness. This

information is not only important to health-care decision makers but also to health-care innovators who can use such information as an input into the dynamics of medical innovation.

HTA is applicable to all technologies

This chapter has demonstrated that the production of evidence was biased towards a marketable technology like PET, rather than a service technology like a stroke unit. HTA can have (and has had) a role in bridging this bias, and bringing evidence (as well as gaps in evidence) to the forefront of the research and policy debate. Similarly, it has a role in assessing older technologies: firstly, so that the relative impact of new technologies can be measured, and also to overcome the lack of evidence on the effectiveness and efficiency of some current aspects of medical practice.

Development of methodology

In refining HTA methodology, HTA practitioners should consider developing and using more consistent approaches for incorporating, for instance, patient values and preferences in the overall assessment.

Diversity in the production and delivery of HTA

Whilst there is widespread international agreement on the definition of HTA, its production varies considerably among countries. This is to be expected. HTA is a product of the individual characteristics of the health-care system in which it is developed. Hence, just as health-care systems differ, so does HTA (Banta, 2003). In fact, the adaptation of HTA to the national health-care system brings advantages. That is, nationally adapted HTA is more likely to play into the right decision-making nodes of the health-care system, and enable the interaction between scientific evidence and national (or even local) values.

Identify where international co-operation on certain aspects of the HTA can be optimised

There is consensus that the part of HTA dealing with appraisal processes should only be undertaken within national and local contexts. Nevertheless, there may be efficiencies in other parts of the HTA process, specifically around technology assessment. Greater international collaboration when it comes to the synthesis of evidence may generate savings and reduce duplication and also raise important methodological issues around transferability (see Chapter 6). After all, many HTA practitioners use the same sources of evidence for their reviews. The International Network of Agencies of Health Technology Assessment has achieved a great deal through its joint evaluations, database and development of an HTA checklist, but there is recognised need for further collaboration in this area. Countries that do not have extensive HTA programmes may especially benefit from such collaboration.

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Chapter 4

DECISION MAKING AND IMPLEMENTATION: AN ANALYSIS OF SURVEY RESULTS

Health-care decisions have an important impact on the use and uptake of health technologies in OECD countries. This chapter presents results from the OECD survey on health technologies, which focused on decision-making processes and implementation methods in survey countries. It considers the policy tools that countries use to implement evidence-based decision making and reports on the main impediments and facilitators within the health-care system for implementing decisions. The chapter also describes how various countries have successfully integrated technologies into their health systems in line with evidence and health system objectives.

Introduction

OECD countries recognise the importance of providing health-care decision makers with high-quality evidence to enable them to make informed choices about the diffusion and use of new and emerging health technologies. Evidence, such as that provided by health technology assessment (HTA), provides decision makers with information on the likely impact that new technologies may have on our ability to prevent, treat and manage disease.

However, access to high-quality evidence is a necessary but not a sufficient condition to integrate technologies into the health-care system effectively. It is increasingly recognised that the successful use of evidence depends, in large part, on the decision-making process. Furthermore, the health-care system itself can support (or impede) evidence-based decision making. The institutional, organisational, political and cultural dynamics of the health-care system all contribute to the policy maker's ability to integrate health-care technologies successfully.

This chapter of the report examines features of the decision-making process which lead to the use and uptake of health technologies in OECD countries. It considers the policy tools that countries use to implement evidence-based decision making and reports on the main impediments and facilitators within the health-care system for implementing decisions. The chapter also describes how various countries have successfully integrated technologies into their health system, in line with evidence and health system objectives.

The OECD health technology survey sought information - provided by decision makers, or people with knowledge of the decision-making process - on tangible case study technologies on the topics of:

- How decisions about health technologies are made.
- How evidence, and in particular HTA, is used in that decision.
- How decisions are implemented.
- What features of the health-care system might facilitate or impede decision implementation.

The survey focused on the decision-making processes in 12 OECD countries[1] for five case study technologies: positron emission tomography (PET), hepatitis C genotyping and viral load testing, telemedicine, prostate cancer screening and stroke technologies (*e.g.* stroke units or stroke services).

Decision making in any health-care system is a complex set of interactions amongst a wide array of players. Previous studies have categorised the health-care system using three broad levels:

- Macro (where decisions are made at national, provincial or insurance company level).
- Meso (where decisions are made at regional health authority or hospital level).
- Micro (where decisions are made at provider or patient level).

The survey addressed decision making at the macro and meso levels[2]. In particular, the analysis focused on:

- *Funding/coverage decisions:* the decision to fund/cover a product or service on either private or public insurance schemes. Such decisions typically cover recurrent expenditures.
- *Investing/planning:* the decision to purchase a technology, including health-care planning decisions. These decisions may involve capital expenditures for technologies such as PET and telemedicine.

The discussion of the survey results reflects the consultations with survey respondents, experts and stakeholders. Telephone interviews were conducted with a number of survey respondents to clarify or provide additional information on survey answers.

Features of decision-making processes

This section presents some of the survey results relating to characteristics of decision-making processes in survey countries. It will show where in the health-care system decisions take place, who was involved, and the respondents' perceptions of *reasonableness* (discussed later in this chapter) in the decision-making process.

1. Undertaken in Austria, Australia, France, Germany, Ireland, Japan, Mexico, the Netherlands, Norway, Spain and Switzerland. Telephone interviews were conducted in all survey countries, as well as Canada, to supplement survey responses.
2. The survey was carried out in countries that have extensive public health-care systems, and as such the results primarily reflect public decision-making processes. However, the survey may still provide important insights into decision-making processes for private health-care insurers and providers.

Where are decisions taken?

Decisions regarding the uptake and diffusion of technology take place throughout the health-care system, with some decisions taking place at the provider level, some at a regional level and others at a national or state level. OECD countries differ markedly on where decisions take place: some are more centralised and others have devolved decision making to regional authorities, insurance companies or providers. The level of decision making is an important element of the decision-making process. Each level is likely to involve different perspectives, responsibilities and incentives. The survey examined where in the health-care system macro and meso decisions about technology uptake and diffusion take place.

The survey showed that decisions are taken at multiple levels (macro, meso and micro) in the health-care system, depending on the roles and responsibilities of decision makers at each level. No clear patterns emerge across OECD countries about what types of decisions take place and where they are made. Instead, these appear reflect the historical and institutional characteristics of OECD health-care systems. Figure 4.1 indicates where investment decisions are made for PET and telemedicine[3] in ten OECD countries. In the case of PET, which has high capital costs[4], decisions take place primarily at the national and state/provincial government level, although investment decisions are also made at the provider level. As might be expected, given the lower capital costs involved, decisions on telemedicine are made more commonly at the regional health authority and hospital level.

Figure 4.1 also shows that providers (in the case of PET), and hospitals and regional health authorities (in the case of telemedicine) play an important role in investment decisions. This may reflect the underlying trend in health policy to make decisions at the local level - closer to patient needs and wants.

The multiple levels of decision making for both PET and telemedicine perhaps indicate the complexities of these two technologies. For instance, PET requires a substantial capital investment and operating budget as well as a degree of co-operation and co-ordination amongst hospitals to share PET facilities efficiently. These features may provide a justification for the involvement of higher level decision makers, including national and state/provincial governments and/or public or private insurers.

In the case of telemedicine, various decision-making levels might be involved because the value of the technology is, in part, based on the way it connects the health-care system. For example, with each additional telemedicine unit, all the existing users of the telemedicine network will benefit because of the additional provision of professional skills[5]. The true value of the entire network may not be taken into consideration by local decision makers, who might be more concerned with the costs and benefits achieved at the local level (rather than a system-wide level). Higher level decision makers might be better placed to consider the wider value of such technologies.

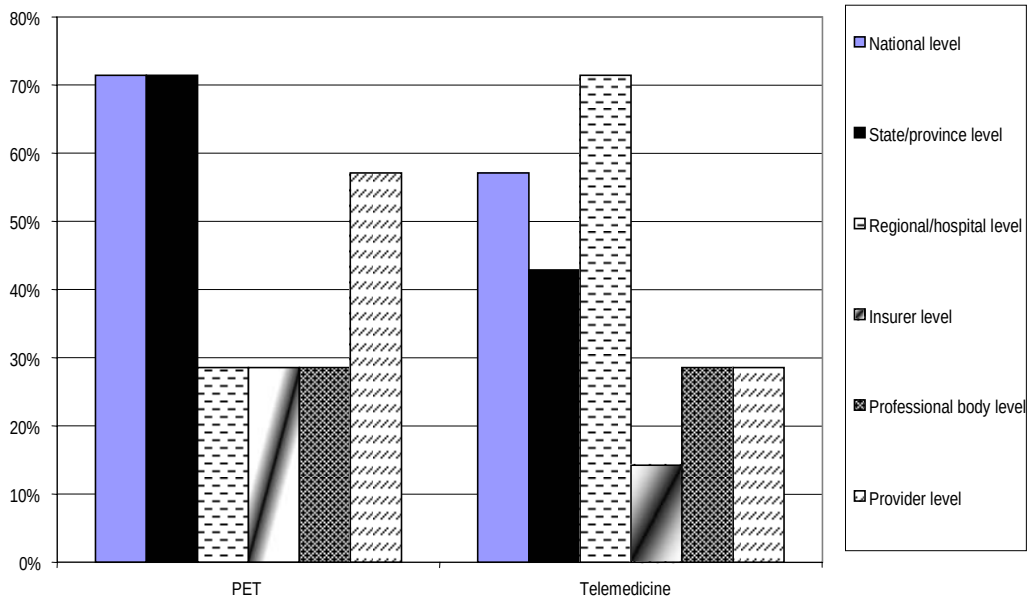
3. These two case study technologies are focused on here because they involve important investment decisions; they have upfront costs, service implications, and are also illustrative of the different types of challenges that decision makers face.

4. Estimated to be EUR 1.9 million plus further costs for layout work (CEDIT, 2002).

5. Analogous to the telephone network or the World Wide Web. The more users and more information available, the higher the value of the network.

The fact that investment decisions on PET and telemedicine take place at a number of levels reveals the strengths and weaknesses that different levels of decision making bring to the process. These strengths and weaknesses may vary according to the specific characteristics of the health technology in question. Local decision makers, for instance, might be better placed to consider the suitability of the technology to local patient needs, locally available professional skills, and local infrastructure. On the other hand, higher level decision makers might be better placed to consider efficient co-ordination and compatibility of health technologies used throughout the health system. These could be important considerations in making effective decisions about health technology.

Figure 4.1. **Where investment decisions are made**



Interviews with survey respondents revealed that decision-making processes are not always clear cut. Several interviewees indicated that it was unclear to them how and where decisions take place. For example, one respondent working in an academic hospital mentioned that the decision-making process varies from year to year and depends on the overall budgetary situation and the pressures on that budget.

A lack (or a perceived lack) of a decision-making process can create barriers to the efficient uptake of technologies. Firstly, it creates doubts over the legitimacy of decisions and may therefore be less likely to be supported by stakeholders. Secondly, incorporating evidence into ill-defined decision-making processes is more complex as the producers of evidence will be less likely to deliver timely and relevant advice.

Who is involved in decisions?

Table 4.1 gives details of the stakeholders who were involved in funding and investment/planning decisions. The table shows that from the sample of case studies and countries in this survey, health-care managers, academics/technical experts and government officials were the most common groups of people involved in both types of decision making. Patients and consumer groups were the least involved in decision making (n=61).

Table 4.1. **Participants in decision making**

	Type of decision	
	Funding/coverage	Investment/planning
Government officials	67%	54%
Industry representatives	21%	25%
Provider groups	33%	43%
Patients/ consumer groups	15%	4%
Politicians	24%	29%
Health-care managers	76%	79%
Academics/technical experts	67%	71%
Others	12%	14%

Some respondents mentioned that patient perspectives were taken into account indirectly through measures such as safety, effectiveness, health-care needs and quality of life. However, such measures may not encapsulate important broader patient values (or perspectives), such as a preference over the type of treatment (rather than just the outcome of treatment).

This is an important issue in terms of health outcomes and costs. For example, several studies have noted that non-compliance with medication regimens is extensive. Studies show that between 5% and 10% of hospital admissions are due to non-compliance, leading to substantial adverse health outcomes as well as additional hospital costs, estimated to be in the range of 0.8% and 1.7% of total health-care expenditures (Cleemput *et al*., 2002). One important determinant of non-compliance arises when there are unpleasant side-effects for the patient from taking medication. These factors can make an important difference in estimating the ultimate effectiveness and costs-effectiveness of intervention. Measures of patient preferences can play a substantial role in assessment and appraisal of new technologies, and may therefore provide useful insights into the value of the technologies.

Many OECD countries have started to recognise the importance of including patient or citizen values in decision making. For example the UK's National Institute for Clinical Excellence (NICE) established a Citizens Council to gain an appreciation of public perspectives on key issues that inform the development of NICE guidance to the National Health Service on the use of treatments and the nature of care (Dillon, 2003).

What choices do decision makers have?

The survey asked respondents to identify the types of choices that decision makers have. For example, can they only say "yes" or "no" in their decision to publicly fund a certain technology, or are there other options?

The survey found that in 38% of cases, decision makers could only make a "yes" or "no" decision regarding the public funding of technologies. In 12% of cases, decision makers could approve the use of technology but then make a separate decision about public funding. In 50% of cases, survey respondents indicated that decision makers could

add conditions to their approval. For example, in some countries decision makers can place a time, geographic or patient group limitation on their decision.

Conditional approvals appear to be an important feature of the decision-making process, especially if there is uncertainty around important decision parameters typically found in new and emerging technologies. Allowing decision makers to opt for conditional approval enables the use of the technology in a limited capacity so that uncertainty is minimised and/or additional information can be gathered.

More countries are making use of such conditional approvals. In Switzerland, for example, hepatitis C genotyping tests have been granted a five-year approval. During this time, the manufacturers of the tests, regulators and diagnostic laboratories have to collect and review additional data, to help overcome key uncertainties. Rigorous procedures have been agreed upon that will provide information to the regulators as well as R&D information to the manufacturers of the test. Similarly, in Australia the funding of PET scanners and services was recommended on a limited scale so that additional data could be gathered to inform future decisions.

Box 4.1. Conditional approval and proposed changes to pharmaceutical policy in the Netherlands

A number of OECD countries are moving away from an era where public health policy was focused on cost containment, and towards an era of accountability, where measurement, value for money and evidence-based practice and policy are key drivers of the reform process.

In the Netherlands, the planned implementation of a new pharmaceutical policy in 2005 provides a good example of conditional decision making. The policy will firstly introduce mandatory use of pharmaco-economic guidelines and secondly provide the opportunity for manufacturers to claim a premium price for innovative drugs.

The third pillar of the new policy introduces the notion of reassessment for new innovative drugs once they have become part of the insurance package. For every positive decision to reimburse a new innovative drug there will continue to be post-reimbursement assessment to evaluate whether claims on therapeutic usage, effectiveness and cost-effectiveness can be validated using "real world" data. Hence, the initial decision to list a new premium price drug is based on cost efficacy (data from trials). However, subsequent decisions will be based on cost-effectiveness data (from real-world experience).

The aim of the policy is to encourage clinical practice based on emerging national evidence and to facilitate risk sharing between payers and providers/producers. The risk sharing component of the policy will be based on agreements between stakeholders on financial caps, volume, market share and cost-effectiveness (Rutten, 2003).

However, for conditional approval to be an effective means of dealing with new and emerging technologies, two criteria need to be satisfied. Firstly, further gathering of data is essential. This requires not only resources but also a commitment from stakeholders to agree to a procedure to collect a minimum data set that will provide additional data around the key decision-making criteria. In the case of the Dutch pharmaceutical policy, post-decision evaluation will rely on observational data and outcomes research using existing data sources. In the case of PET in Australia and hepatitis C genotyping in Switzerland, new data procedures had to be agreed to and new data gathered.

Secondly, conditional approval can only work if decision makers can, upon receiving new information, re-evaluate their decision, not just in theory but also in practice. This would appear to require a commitment from all stakeholders to agree to the process and

commit to the final outcome of the decision. Risk-sharing agreements between producers of technology and public authorities can also become part of the conditional approval process. This is discussed further in this chapter under the heading "innovative approaches to managing decision implementation and technology diffusion".

The use of conditional approval mechanism enables early access to the technology, recognise uncertainty in the evidence-base and can also contain risk-sharing mechanisms. Such mechanisms also enables decision makers to take into account the value of innovation, based on real-life data. The use of conditional approval mechanisms is yet to be fully evaluated but they do offer some prospect of better integration of new health technologies into health-care delivery.

Reasonableness in decision making

The "accountability for reasonableness" framework was developed by Daniels and Sabin (1997) to evaluate whether priority-setting decisions could be considered "fair". The framework has four conditions: relevance, transparency, appeals and enforcement. They are described in more detail in Table 4.2. This framework has been used in a number of recent studies, including priority setting analysis in Canada and Norway. It has also been recognised internationally as an appropriate ethical framework for evaluation priority setting in health-care institutions (Martin, *et al.* 2003).

Table 4.2. **Conditions for reasonable decision making**

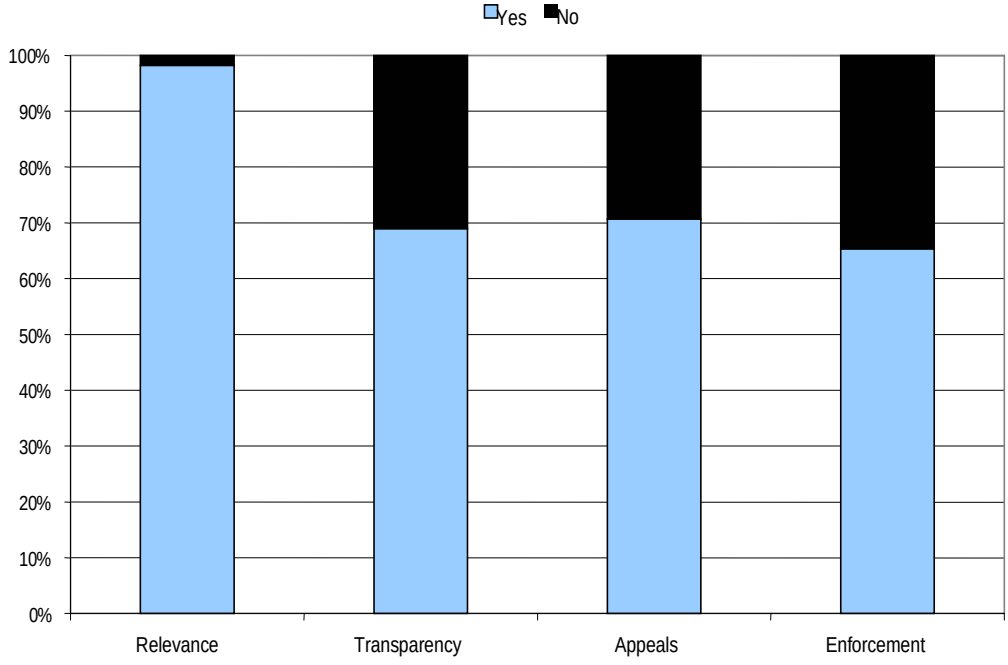
Condition	Description
Relevance	Decisions must rest on reasons (including information and principles) that fair minded parties can agree are relevant to meeting context specific need under resource constraints. Fair minded parties are stakeholders who are predisposed to decision making according to rules of mutual co-operation.
Transparency	Publicly accessible information was available with respect to the decision-making process, the evidence used and the rationale for the decisions.
Appeals	The process must include a mechanism for challenge and dispute resolution regarding priority-setting decisions.
Enforcement	There must be voluntary or public regulation to ensure that the first three conditions are met.

Source: Adapted from Martin *et al.* (2003).

The OECD health technology survey asked respondents to provide their views on whether the decision-making process for the case studies in their countries met the four conditions listed in Table 4.2. The survey asked eight questions on whether the decision-making process met the four conditions of "reasonableness". Depending on the number of conditions met, the decision-making process was classified as exhibiting either a high or low level of "reasonableness". Figure 4.2 shows the percentage of conditions that respondents thought their decision process met (n=58). Almost all respondents thought that their decision was "relevant" (*i.e.* based on reason, evidence and principles).

However respondents were less certain about whether their decision-making process met the remaining three conditions of reasonableness. For example, respondents reported that in 31% of cases there was a lack of transparency about the decision-making process and in 30% of cases there was no appeals mechanism.

Figure 4.2. **Meeting the conditions for "reasonable" decision making**



Decision acceptance by stakeholders

The survey asked respondents to indicate whether they thought that stakeholders in the decision outcome accepted the decision made. Overall, 64% of respondents said that stakeholders accepted the decision most of the time and 36% reported that stakeholders accepted the decision "some of the time" or "hardly ever". Survey respondents also indicated that acceptance amongst stakeholders of the decision-making process is an important contributing factor in gaining broader support for decisions and therefore improving the ease with which decisions can be implemented.

Table 4.3 links the degree of reasonableness in the decision-making process with the level of acceptance of decisions by stakeholders. It shows that where decision-making processes exhibited a high level of reasonableness, stakeholders accepted the decision most of the time. However, for decision-making processes where the level of reasonableness was low, there was a decline in the level of stakeholder acceptance (n=59).

Table 4.3. **Level of acceptance by level of reasonableness in decision-making process**

Level of reasonableness	Acceptance of decisions by stakeholders	
	Most of the time	Some of the time or hardly ever
High	83%	17%
Low	37%	63%

The survey results support the notion that the degree of "reasonableness" may be influential in bringing more widespread acceptance of the decision. This is likely to be an important contributing factor when it comes to implementing decisions successfully.

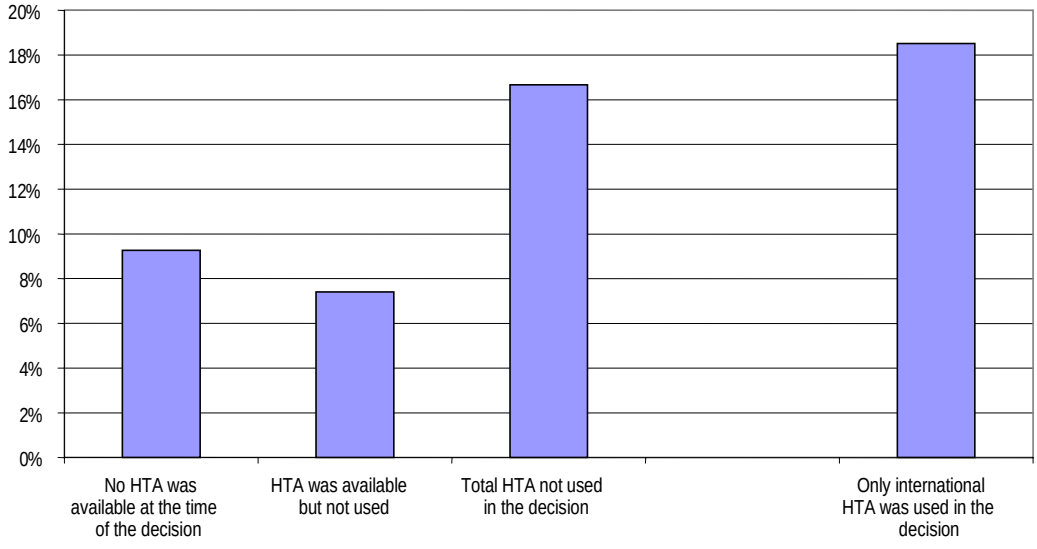
The analysis of survey data also examined whether the number and type of stakeholders involved in the decision-making process were related to broader stakeholder acceptance of decisions. The quantitative survey results did not find any evidence for (or against) this idea. However, qualitatively a number of survey respondents indicated the importance of broader stakeholder involvement in successful decision making and decision implementation.

What evidence was used in decision making?

Survey respondents were asked about the value, availability and use of HTA to support decision making. Respondents were also asked whether the HTA used in the decision was produced domestically or internationally. Overwhelmingly, survey respondents thought that HTA was of high value to the decision-making process and that it met their expectations.

Figure 4.3 (n=54) reveals that in 19% of cases, only international HTA was used to support decisions. In 10% of cases, no HTA was available and in 7% of cases HTA was available but not used in the decision on the case study technologies. Thus, in total, no HTA was used in 17% of cases. This of course does not mean that no evidence was used. For example, a recent report by Lehoux *et al.* (2003) found that Canadian decision makers use a range of information sources besides HTA.

Figure 4.3. **The use of HTA in decision making**



The OECD survey also asked respondents whether they thought the process of making decisions about the uptake of technologies was a "formal" one (defined as a regulatory or administrative requirement to make a decision) or an "informal" one (where there was no such requirement). Eighty per cent of respondents (n=69) said that decision making in their country for the case study technologies was formal.

The survey results provide some support for the notion that a more formal decision-making process is more likely to use HTA to support a decision. Over 85% of decisions taken in the more formal process drew on HTA. This figure dropped to 60% for "informal" decision making – although the sample size for the number of informal decision was small, accounting for only 20% of the sample (n=69).

Nevertheless, it would seem plausible that the existence of a decision-making structure enhances incorporation of evidence and HTA. In part, this may be because HTA practitioners are able to direct their assessments to pre-defined target groups who are responsible for formal decisions.

In several OECD countries, policy makers are working on developing processes to enhance the use of HTA to support decision making. In Ontario, Canada, for example, the Health Technology Advisory Committee brings together senior hospital decision makers and clinical experts to identify new and emerging technologies. This body sets priorities for assessment, which are conducted under the auspices of the provincial Ministry of Health and Long Term Care. The Ontario model systematically incorporates evidence into decision making amongst a group of hospitals within the same health system that face similar concerns and patient needs. The Committee has created a process whereby local decision makers drive the agenda for HTA, and at the same time improve the likelihood that HTA will actually be used.

The policy in Ontario is intended to ensure that evidence is examined along the entire development cycle of the technology. It enables better use and dissemination of field evaluations of new (non-drug) technologies to provide needed evidence for decision making. It also enables an early assessment or evaluation of a technology that is based on the specific questions that decision makers have and is aligned to their needs.

Decision-making needs and HTA content

An important issue for generating better use of HTA in decision making is to ensure that the topics covered in HTA match the needs of decision makers[6]. HTA is intended to assess the broad impacts that technology has on, for example, health outcomes and cost-effectiveness, as well as social, ethical and legal considerations. However, in practical terms, and as shown in Chapter 3, HTA usually covers a more narrow range of topics and does not always fulfil the information requirements for decision makers.

Table 4.4 illustrates the extent to which HTA meets the needs of the decision-making process according to survey respondents. Key topic areas that were identified as user needs are listed in the first column. The second column shows the percentage of HTAs in the survey sample that contained information on the list of topics displayed in the first column. The third and fourth columns reveal the percentage of survey respondents who thought each topic was important or very important in reaching a decision.

6. The decision makers' needs are itself an area where there is potential for value-loading or group-think. The assumption is made in this section that "needs" are rationally and widely based, but it is accepted that in principle this may not always be the case.

The results in Table 4.4 demonstrate that in most cases HTA content matches the perceived needs of decision makers in terms of information on effectiveness, quality and disease severity. However there are also some important differences between what decision makers require and what some HTAs offer. For example, only 42% of HTAs contained information on equity, yet 82% and 78% of decision makers thought that this was an important topic in their funding and investment decisions respectively. Similarly, only 40% of HTAs covered organisational and professional implications, yet 80% and 95% of decision makers thought that this was an important topic in their funding and investment decisions respectively. Table 4.4 also illustrates differences amongst decision makers in the importance placed on specific topics listed in the first column. In general, HTA topics such as organisation, cost-effectiveness, total costs/savings were considered to be more important by those respondents involved in investment decisions than those involved in funding decisions.

Table 4.4 illustrates the need for a better dialogue between the producers and users of HTA. A dialogue that occurs early on in the assessment process is likely to achieve better alignment of HTA content, decision makers' needs and policy issues.

Table 4.4. **HTA content and decision makers' needs**

Topic	**Percentage of HTAs that contain information on the topic (n=27) %**	**Percentage of respondents who considered this topic important or very important in the decision**	
		Funding decisions (n=28) %	Investment decisions (n=27) %
Efficacy/effectiveness	96	86	95
Quality/safety	89	91	91
Psychological and ethical considerations	40	67	75
Organisational and professional implications	65	80	95
Cost-effectiveness	74	74	95
Total costs/savings	63	70	91
Burden of disease	69	81	82
Disease severity	68	71	67
Equity	42	82	78
Social benefits	62	63	71
Patient preferences	41	57	64
Industry/R&D	19	38	58
Waiting times	36	50	59
Lack of alternative treatment	24	48	60
Overall HTA recommendation		67	69
Political considerations		55	57
Media attentions		39	50

Implementing decisions

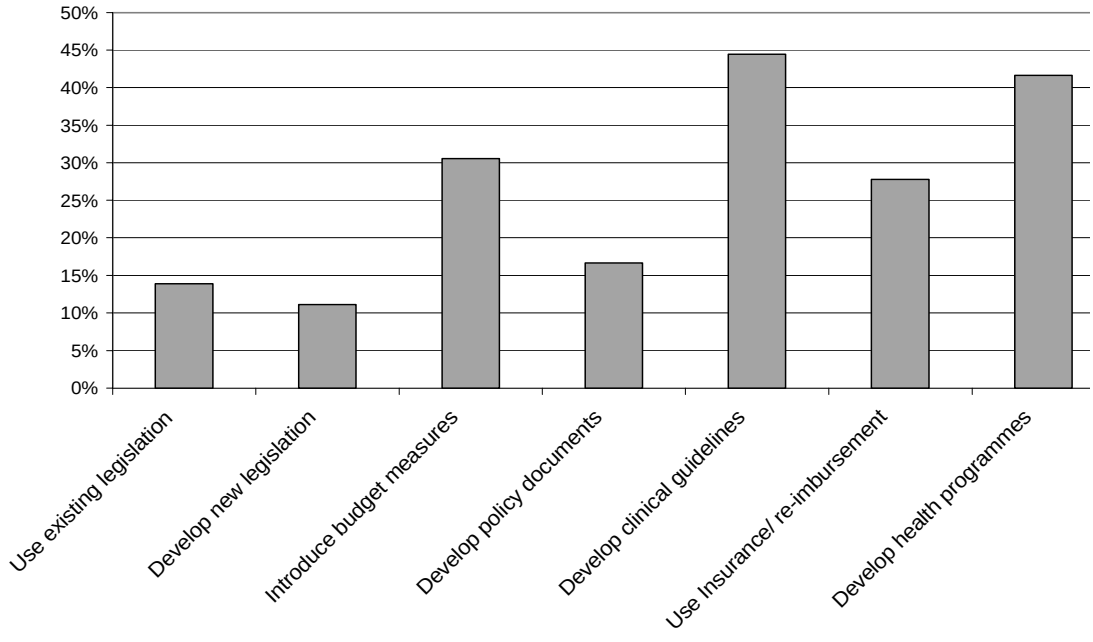
Successfully implementing decisions about technology availability and coverage is a key challenge for many health-policy makers. The methods used for implementing decisions are a key factor in determining whether they will actually influence practice and, through it, health outcomes.

This section provides an overview of the way OECD survey countries implement decisions at the macro and meso levels. For the purposes of this project, the implementation of decisions was considered in two parts. The first part considered the transformation from decision to policy. That is, it examined what type of policy instruments survey countries used to implement decisions. The second part considered how the policy instrument was then introduced into the health-care system to try and influence micro level decision making at the clinical practice level. Figure 4.4 presents the findings of the first part and Figure 4.5 the second part. This section of the report also takes a more detailed look at the way some countries have implemented decisions with regard to some of the case study technologies. It also examines innovative approaches to implementation used more broadly in OECD health-care systems.

Overview of decision implementation

As demonstrated in Chart 8 (n=36) decision makers have a range of options at their disposal to implement their decisions. Nearly 45% of respondents mentioned that clinical guidelines or recommendations were developed to implement decisions. The development of specific health programmes was also a common method of implementation.

Figure 4.4. **How were decisions translated into policy?**

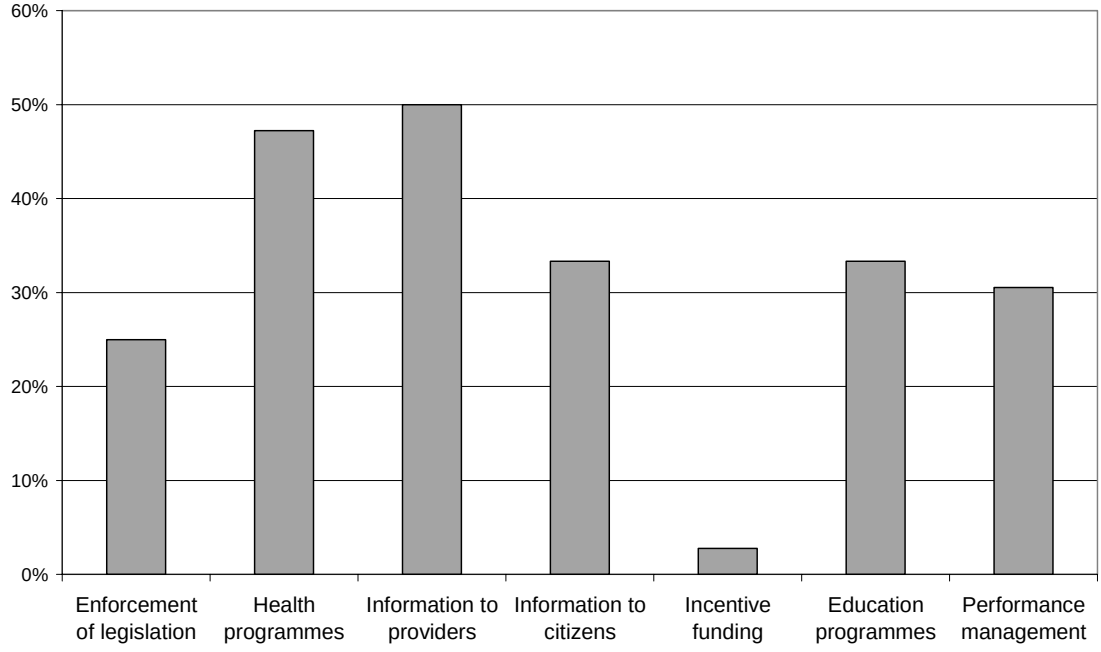


The OECD survey also asked respondents to comment on how policy instruments were put into practice. Figure 4.5 (n=36) shows the results. Direct health-care programmes and provision of information to health-care providers were the common means by which policies were implemented to inform and influence practice. Policy makers also use education programmes (for example ongoing education programmes for health-care providers) aimed at implementing policy decisions in clinical practice. Incentive funding, to try and influence practice, was used in a small minority of cases.

Evidence on the impact of policy instruments on clinical practice is limited in many OECD countries. More evidence of the effectiveness of these policy instruments can inform decision makers at macro and meso levels to choose what policy instrument to use to influence clinical practice.

The current level of variation in technology diffusion (see, for example, Figure 1.1 in Chapter 1) and uptake amongst OECD countries suggests that there can be better alignment of evidence, policy and practice. This suggests a push and pull role for policy makers and producers of HTA. HTA producers can push HTA messages by ensuring that their findings are relevant to the decision makers' needs, as well as authoritative and widely available. Policy makers, on the other hand, have a responsibility to facilitate the use of HTA in decision making and policy instruments, and inform HTA producers of their needs. This can be achieved through better education programmes in the use of HTA and improved links between producers and users of HTA

Figure 4.5. **How was policy put into (clinical) practice?**



Implementing negative decisions

After having considered and appraised the available evidence, decision makers may of course decide against implementation of a technology. The possible reasons for such decisions vary but could be made on the basis of evidence against the effectiveness or cost-effectiveness of the technology, or a lack of evidence. If a decision is negative, there may still be a need to take active steps to implement that decision. Canada and Norway provide two case studies of the way they implemented negative decisions.

In all survey countries assessments concluded that population-wide prostate cancer screening would not be appropriate. No survey country has thus far instigated a prostate cancer screening programme, although many countries allow prostate cancer screening to take place in, for example, physicians' rooms. The recommendation not to implement a population-wide prostate cancer screening programme was primarily based on a "lack of evidence of benefit" rather than "evidence of negative impact" (hence the start of randomised control trials such as the European Randomized Study of Screening for Prostate Cancer [ERSPC] in 1994).

Whilst there is considerable agreement that there is a lack of evidence on the benefits of population-wide prostate cancer screening, the rate of screening in physicians' rooms, particularly prostate specific antigen (PSA) tests, is thought to be increasing. Although international comparative numbers are scarce, Australian statistics show that (even without a screening programme as such) over 800 000 PSA tests were conducted in 2002 (population of 3.3 million men aged 45 and over) (AIHW, 2003; HIC, 2003). In Germany, the use of the PSA test almost doubled between 1990 and 1995, from 783 000 to 1 533 400 (population of 13.7 million men aged 50 or over) (Perleth, 2001).

In a bid to ensure that PSA testing is conducted appropriately and in a targeted fashion, decision makers, HTA practitioners and physician groups came together in Quebec to develop an informed consent form, which provides full information to individuals on PSA testing and treatment of prostate cancer. Individuals wanting to undertake the test must sign this form in recognition that they have read and understood the information provided to them.

Similarly, a Norwegian HTA working group on the use of thrombolytic therapy recommended against widespread use of this therapy for acute ischemic stroke patients. The working group found that the evidence on thrombolytic therapy is uncertain and that there is a risk of intercranial haemorrhaging as well as inappropriate administration of the medication. The working group's decision was directly disseminated to health-care providers and considered to have been successful in limiting the use of thrombolytic therapy for acute ischemic stroke patients. One element considered to be part of the success was that the working group's membership consisted of clinical leaders in the field of stroke care in Norway.

Experiences from Norway and Quebec illustrate that support amongst opinion leaders and clinicians can be important in affecting the subsequent diffusion of technology in line with local HTA recommendations and decisions.

Policy instruments used to implement decisions on PET and stroke units/services

The range of policy instruments available to manage the implementation of decisions of course depends on the specific technologies in question. Tables 4.5 and 4.6 illustrate the types of mechanisms that survey countries have available to them to implement decisions on PET and stroke technologies respectively. Countries have more mechanisms to implement decisions and manage diffusion for PET than for stroke technologies. For example, more countries have the option of including (or excluding) PET services in public reimbursement programmes. They can also use pre-market controls for PET.

Most survey countries have multiple mechanisms available to them to implement PET decisions. By contrast, countries rely almost solely on planning tools to implement stroke technologies, although some have performance management type mechanisms available, and Mexico has the ability to include (or exclude) stroke units in public reimbursement programmes.

Table 4.5. **Available policy mechanisms to manage diffusion of PET**

	Pre-market controls	Exclusion from public reimbursement	Planning tools for specific technologies	Incentive payments for institutions	Incentive payments to providers	Encourage competition	Medical audit and reviews
Australia	√	√	√				√
Austria	√	√	√				√
France	√		√	√	√		√
Germany		√	√				
Ireland	√						√
Japan	√	√					
Mexico			√				
Netherlands[7]			√	√	√		
Norway		√	√				√
Spain							√
Switzerland	√	√	√				

7. In the Netherlands, the Specific Medical Procedures Act (Section 2) enables the government to restrict technology diffusion for reasons of efficiency and quality. The Act is currently used for ten types of technology (*e.g.* specific neurosurgery, radiation therapy, heart surgery, various transplantations, intensive care for neonates, IVF and clinical genetics). The Act was not used in the case of PET technologies.

Table 4.6. **Available policy mechanisms to manage diffusion of stroke technologies**

	Exclusion from public reimbursement	Planning tools for specific technologies	Incentive payments for institutions	Incentive payments to providers	Encourage competition	Medical audit and reviews
France		√				
Germany		√				
Mexico	√	√				√
Netherlands		√				√
Switzerland		√				√

Table 4.7 shows which countries have provided ear-marked public funding to introduce PET. Five countries provided additional capital funding to cover (at least in part) the capital costs incurred by health-care institutions in purchasing PET machines. In France, public funding will be made available in 2004 to six regions to assist in the purchase of PET machines, as part of the national cancer plan. Four countries provide additional recurrent funding for PET services.

Table 4.7. **Public financial assistance to health-care institutions for PET**

	Capital funding	Recurrent funding
Australia	√	√
Austria	√	
France	√	√
Germany[8]	√	√
Ireland		
Japan		
Mexico		
Netherlands		
Norway	√	
Spain		
Switzerland	√	√

Table 4.8 indicates which countries have provided additional public funding to introduce stroke units. Here only two countries have provided additional capital and recurrent funding. In Germany and Australia (New South Wales), state or local government authorities have distributed funds to hospitals to introduce stroke units and deliver key service aspects associated with better stroke patient care, such as the continuous availability of computed tomography scanners for stroke patients. In the

8. Germany has provided additional financial assistance for the purchase and recurrent costs of PET machines in hospitals only.

Netherlands, whilst the establishment of stroke services had to be met out of the existing budget, additional funding was made available to the task force in charge of the stroke service implementation programme.

Table 4.8. **Public financial assistance to health-care institutions for stroke**

	Capital funding	Recurrent funding
Australia (NSW)	√	√
France		
Germany	√	√
Mexico		
Netherlands		
Norway		
Spain		
Switzerland		

A number of OECD countries have recognised the importance of establishing implementation programmes to help enhance evidence-based policy and practice, especially for technologies where there are wider service implications.

Implementing decisions: barriers and facilitators

Implementation programmes can not be designed without due consideration of the wider health system influence on clinical behaviour. The institutional and financial aspects of the health system can have an important influence on whether decisions will be successfully implemented. Some aspects of the health system will serve to facilitate decision implementation, whereas other factors may impede implementation.

The OECD survey asked respondents to indicate what features of their health system they considered important in facilitating or impeding the implementation of decisions. Table 4.9 (n=35) shows the results for facilitating factors. Seventy-four per cent of respondents thought that having a trusted source of evidence on which to base decisions was important or very important. Having some additional funding to implement recommendations was thought to be important (or very important) to 88% of respondents.

This finding suggests that when no additional funding is available to implement technologies (and implementation has to be met out of the existing budget), there is an incentive to take up technologies that are cost saving. At the same time, this creates disincentives to take up technologies that can increase costs, regardless of how effective the technologies may be.

Respondents also thought that having the flexibility to move resources from one budget to another was important or very important (63% of respondents thought so). This is a key issue that relates to the issue of "silo" funding – where decision makers have responsibility over a budget that is for funding a defined set of health inputs (such as pharmaceuticals).

Table 4.9. **Health system factors that facilitate decision implementation**

	Not important	Somewhat important	Important	Very important
Evidence comes from a trusted source	14%	11%	34%	40%
Flexibility in health-care budgets	14%	23%	34%	29%
Funding for implementation is available	6%	6%	35%	53%
Direct benefits to my department	26%	9%	37%	29%
Payment mechanisms encourage uptake	29%	26%	24%	21%
Performance management systems	15%	18%	48%	18%
Stakeholders are involved and support decisions	11%	34%	14%	40%

Silo funding may create disincentives to efficient decision making. Decision makers responsible for a silo budget may narrowly focus on the technology's cost impact in terms of their budget only, potentially ignoring the value, costs and savings in other health-care budgets or more widely. This can lead to a situation in which cost-effective technologies are potentially under used or cost-ineffective technologies over used.

Table 4.10 shows respondents' thoughts on what they perceived to be the main barriers to decision implementation. Their answers on the lack of flexibility of budgets were consistent with the results in Table 4.9. Sixty-three per cent of respondents thought that not having additional funding was an important or very important barrier to implementation.

Fifty-seven per cent of respondents thought that the payment mechanism in their health-care system discouraged decision implementation. As demonstrated by Table 4.9, 45% of respondents thought that their health-care system's payment mechanism played an important role in encouraging uptake of policies. These findings are backed by comments from a number of survey respondents which highlighted instances where payment mechanisms created disincentives to align practice with evidence. The survey results suggest that decisions can be implemented more effectively when provider payment mechanisms are better aligned with evidence-based decision making.

Table 4.10. **Health system barriers to decision implementation**

	Not important	Somewhat important	Important	Very important
No flexibility in moving resources between budgets	14%	23%	34%	29%
Resources cannot be freed to adopt new technologies	6%	17%	23%	54%
Payment mechanisms discourage uptake	27%	15%	36%	21%
Too few mechanisms to inform providers about the policy	21%	32%	26%	21%
No direct benefits to my department	34%	17%	20%	29%

Innovative approaches to managing decision implementation and technology diffusion

Many OECD countries are gearing their health systems towards policies that emphasise measurement and accountability. The key drivers of this new era are the concepts of value for money and evidence-based policies and practices. These notions are slowly being implemented (Rutten, 2003).

The new challenge for many OECD policy makers is to develop policy instruments that lead to the optimum levels of diffusion or use. The optimum level is reached when a number of health system goals are taken into account, including safety, effectiveness, value for money, equity and responsiveness.

In recent years, many OECD governments have started to introduce policy tools that aim to achieve multiple health system goals simultaneously. The following set of innovative policy tools, whilst not exhaustive, provides some useful examples of

mechanisms that integrate health technologies in line with multiple goals and the best available evidence.

Cost, volume, and value agreements

Cost, volume and value agreements are commonly used to shift and control costs. Under Australia's fee-for-service Medicare programme, for example, price-volume agreements have been implemented for pathology, diagnostic imaging, and anaesthetics. In effect, the government and professional groups agree that expenditure will be limited to a certain amount over a period of three to five years. If utilisation exceeds expectations, prices go down and vice versa (although the latter has only been a hypothetical possibility to date). When new technologies are approved for reimbursement, total expenditures are allowed to increase; however, the cap is re-fixed at that point. This provides certainty as to outlays, despite the formally uncapped nature of the financing arrangements.

Some agreements aim to take into account the value of technology, but this is an imprecise art. In the United Kingdom, for example, an agreement between government, industry, providers and other stakeholders was reached on the purchase and provision of Beta Interferon in the treatment of multiple sclerosis. Under this scheme, if an agreed level of effectiveness was not achieved, the government and manufacturer would renegotiate the price of the drug. This mechanism can increase cost-effectiveness and promote access to new technology whilst further evidence is gathered. It can also encourage the manufacturer to promote appropriate prescribing. Manufacturers of the drug have an interest in drugs being prescribed to patients who are most likely to benefit most, so that high levels of effectiveness are attained when prices are re-examined.

Payment mechanisms

Payment mechanisms can act as powerful incentives for the diffusion and uptake of technologies. A number of OECD countries have made moves towards blended payment systems that combine elements from other mechanisms such as fee-for-service, capitation payment and salary (Docteur and Oxley, 2003). Such payment systems also offer the opportunity to incorporate evidence in decision making at the provider level. For example, countries can offer bonuses for meeting certain health-care targets or create relatively higher rewards for services (or products) that are highly effective and cost-effective.

Planning agreements

Planning agreements among various stakeholders, including national and provincial governments, can be used to establish diffusion guidelines. Such agreements enable a central agency, for example, to set key objectives and help diffuse technologies in line with these objectives. In Austria, national and regional decision makers have agreed on a plan to expand diffusion of PET machines from its current 1.23 to between three and five per million population. The plan takes into account the level of clinical need, population distribution and geographic equity of access.

Implementation and co-ordination programmes

Implementation programmes appear to be effective in appropriately diffusing technologies where there are wider service implications, as examples from the Netherlands and Norway demonstrate. In the Netherlands, following the successful conclusion of six regional pilot stroke service schemes, the government decided to

commence a national stroke-service implementation programme. The working group in charge of the implementation has subsequently produced guidelines for developing stroke services, as well as a comprehensive strategy for their implementation in 23 regions. The working group is currently involved in the implementation phase, and there are early indications of successful national implementation.

The Norwegian National Centre for Telemedicine is a resource centre that gathers, produces and provides information about telemedicine. It supplies research, development and advisory services to assist the Norwegian Health Service in implementing telemedicine services. It has an active research programme that pilots and evaluates new projects before diffusion. The Centre also examines future technologies, applications, and service models that may bear on future health service delivery.

Such implementation and co-ordination programmes may be especially useful for technologies that can alter the organisation of care.

Co-operation among service providers

Enhancing co-operation between providers and health-care organisations may reduce competitive pricing pressures; but co-operation appears to be associated with lower rates of capital-intensive acquisitions (Pritchard, 2002). It also appears to be a major success factor in multidisciplinary service provision. Examples of such services include hospital-in-the-home programmes and stroke services. These service types require seamless service provision from a wide array of providers found in the ambulatory, acute and community sectors. Co-operation amongst these providers has been found to be vital to the success of such programmes.

Discussion

Results from the survey have demonstrated that OECD countries use a wide range of decision-making practices to make choices about new and emerging health technologies. Some key characteristics of decision making have been examined to determine which of them can lead to more informed choices and better acceptance of decisions amongst stakeholders. The chapter has outlined the views of survey respondents and workshop participants on the main health system features that might enable better decision implementation. It has also discussed their perceptions of some of the main barriers to decision implementation.

Based on the results, and a discussion of the results at the workshop in the Hague, several observations can be made regarding better decision making, the enhanced use of evidence in decision making, and implementation activities.

Decision-making processes

Decisions are made throughout the health-care system. This study focused on the macro and meso levels, but also examined how these two levels try to influence decision making at the micro level. An important consideration in developing better decision-making processes is to first take into account which level of decision maker is best placed to make a particular decision. An understanding of the decisions that can best be handled at the macro, meso or micro levels can help empower decision makers to make efficient choices. The case study technologies presented in this project reveal that there are important reasons why decision making should sometimes be shared across a variety of

levels. The case studies also reveal that decision-making roles can vary depending on the particular characteristics of the technology.

In a significant number of instances there was no obvious means of identifying any clear process by which decisions regarding the funding, investment and planning of health technologies were taken. The lack of a clear decision-making process, or lack of knowledge about the process, can lead to the absence of transparency and make it more difficult to disseminate the findings of HTA effectively.

The most common groups involved in decision making are health-care managers, government officials and academics or technical experts. Patient and consumer groups were the least likely to be involved in either funding or investment decisions. A number of countries have started to recognise the need to include patients/citizens in their decision-making processes, as well as in priority setting exercises. The UK's National Institute for Clinical Excellence, for example, established a citizens council in 2002.

In an increasing number of countries, decision makers are authorised to approve a technology on a conditional basis, enabling limited access to it on a trial basis. Conditional approval mechanisms allow further gathering of information about the technology to overcome key uncertainties. At the same time, they can minimise many of the risks associated with widespread diffusion of a technology of uncertain value. However, successful use of conditional approval depends on the capacity of decision makers to gather new information and withdraw support for a technology if it is found to be relatively ineffective or inefficient. Just as important is the capacity of decision makers to strengthen and confirm support if the technology is found to be effective or efficient.

There is wide support for the notion that a "reasonable" decision-making process can enhance broader stakeholder support for decisions. Broader stakeholder support is important if decisions are to be successfully implemented.

The use of HTA in decision making

Analysis of the survey results shows that there were a significant number of cases where HTA was not used or not available to decision makers. Even given the well-known case study technologies that were part of this survey, in 17% of cases no HTA was used in decision making. There is also a need for improvement in aligning the needs of decision makers with the content of HTA. Overwhelmingly, survey respondents and interviewees reported that they valued the role of HTA in decision making, but suggested that this role could be enhanced by ensuring that HTA is better adapted to the policy question, the technology, and the needs of decision makers. This means ensuring the timely availability of information, in line with decision priorities, and recognising the various dynamics of different technology markets. Responsibility for achieving a better alignment of HTA with decision makers' needs rests with both the producers and users of HTA. Several (mainly smaller) OECD countries have limited HTA resources. They often rely on international HTAs and are confronted with challenges around transferability of assessments from one setting to another, discussed further in Chapter 6.

The usefulness of HTA appears to be enhanced when the evidence produced can slot into designated decision-making nodes of the health-care system. Furthermore, HTA is more likely to be used if a number of factors are present, such as well-chosen policy instruments, a prior commitment to make use of technology assessment findings, and appropriate technology assessment methodology (Boer, 2003). This in turn suggests that

technology assessment has to be tailor-made for the policy questions and linked more comprehensively with innovation and other aspects of policy making.

Decision implementation

The ease with which decisions can successfully be implemented will depend on the effectiveness of implementation tools and the way the health-care system impedes or facilitates decision implementation.

The use of high-quality and trusted evidence was seen as an important factor in the successful implementation of decisions, as was greater flexibility of resources and assistance in financing the implementation of new technologies. A number of OECD countries have recognised this and established agreements for additional funding to facilitate the purchase and use of new technologies based on health needs, evidence on effectiveness and cost-effectiveness, and equity of access.

Decision makers have a wide range of implementation tools available to them, although these vary in scope depending on the technology. For example, most countries had a range of tools available to implement decisions regarding PET, but far fewer tools were available for stroke units and services. This result reflects the need for the design of implementation tools that are better equipped to deal with the opportunities of specific technologies and the likely (unintended) barriers put up by the institutional rigidities of the wider health system. The issue of designing toolkits and policies for appropriate diffusion of specific technologies is taken up further in Chapter 8, which focuses on new medical advances in the field of biotechnology.

Health-care policy levers need to be better aligned with the best available evidence. This includes developing or setting policy levers that either create incentives, or neutralise disincentives, for decision makers (in all parts of the health-care system) to incorporate evidence into their choices.

"Silo" funding can act as a barrier to the efficient adoption of technologies. Decision makers who are responsible for their own, narrowly defined budget will be reluctant to adopt new technologies that may add to their costs, even though evidence suggests that the technology may be cost-saving overall (*e.g.* by offsets in patient costs and/or sickness benefits). There is a need for a greater understanding of the impact of incentives on efficient purchasing. There is also a need to identify methods which align incentives with evidence and health system objectives.

In recent years, many countries have moved away from blunt supply side controls for technology use and diffusion. In an era that places greater emphasis on value for money, measurement and accountability, OECD countries have developed and used policy tools that aim to achieve multiple health system goals simultaneously. Some of these innovative policy instruments can take into account the value of new technologies but also recognise the uncertainty surrounding medical innovation. OECD countries have also implemented risk-sharing mechanisms to enable early access to safe technologies. Whilst there is a need to evaluate these new mechanisms fully, they offer some prospect for better integration of new health technologies into health-care delivery.

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Chapter 5

THE USE OF HEALTH TECHNOLOGY ASSESSMENT IN THE HEALTH-CARE SYSTEM: LESSONS FROM THREE OECD COUNTRIES

Luis Durán-Arenas
Mexican Institute of Social Security, IMSS
and
Damian Coburn
Department of Health and Ageing, Australia

Health-care systems play an important role in determining how decision makers use the information provided through health technology assessment. This chapter looks at how three OECD health-care systems (Canada, Mexico and Australia) are dealing with the challenge of effectively adapting and incorporating HTA into their respective decision-making structures. Whilst the approaches differ markedly, each country's efforts are anchored to the institutional, organisational and cultural features of the health-care system.

Introduction

As has been pointed out elsewhere in this report, there is significant variation in the way OECD member country health systems have evolved. Historical, political and cultural factors have strongly influenced the institutions and organisation of health care. It should therefore not be surprising that health-care decision-making processes also vary among countries. The extent to which health-care systems are centralised, the degree to which health-care services and products are regulated, and the role of government in the provision of health care, to name just a few, are important differences in health systems and will inevitably impact on decision-making processes.

The use of health technology assessment (HTA) in health-care decision making is also highly variable. However, while there is substantial international agreement over a number of aspects of HTA methodologies, decision-making processes and the use of HTA tend to reflect local circumstances including health needs, health financing and service provision arrangements, policy objectives and the level of influence and control of decision makers themselves.

This chapter examines the important role health-care systems in determining how decision makers actually use the information provided by HTA practitioners. It considers the features of the health-care system that can impact on the functioning of HTA, taking into account some recent empirical research from Canada on how HTA practitioners and users interact. This chapter also looks at efforts in three OECD (Canada, Mexico and Australia) health-care systems to effectively adapt and incorporate HTA in decision making.

HTA functions

The principal functions of HTA have been defined by the International Network of Health Care Technology Agencies (INAHTA) as:

- Technology safety: To assess the potential adverse effects of diagnostic and therapeutic technologies.
- Efficacy and effectiveness: To assess the capacity of technologies to produce beneficial effects, both in ideal and real situations.
- Efficiency (economic evaluation): Once the effectiveness of the technology has been tested, it is necessary to evaluate its efficient application in the system.
- Social consequences (intended and unintended): It is then necessary to assess the social consequences of the application of the technology. This includes the assessment of equity, as well as the opportunity cost associated with the use of technology.
- Ethical implications: To consider the ethical implications of decisions about the incorporation of new technologies (*e.g.* human reproductive cloning).
- Acceptability, availability, accessibility, and utilisation indications: This final step includes the assessment of operative issues about the incorporation and use of medical technology (Banta *et al.*, 1981, Conde, 1999).

It should be noted that not every HTA addresses each of these issues (see Chapters 3 and 4). However, the ultimate aim of HTA is to inform decision making on the basis of the assessment functions listed above, with decision makers ranging from ministers to individual health service providers[1].

HTA within the context of the health-care system

The way HTA is used will to a large degree be determined by the historical and organisational features of the health-care system. For example, in some countries certain decisions are made centrally and HTA is targeted at central decision-making processes. In other countries these decisions are devolved, and HTA messages are disseminated to local decision makers. To be useful to decision makers, HTA must be tailored to the decision nodes of the health-care system and the needs and interests of decision makers at each of those nodes.

For the purposes of the discussion in this chapter, it might be useful to view the health system environment (in which HTA operates) in two dimensions. The first dimension represents the level of regulation in the health-care market, where at one end of the scale, the provision of health is essentially a free market activity and at the other end of the scale it is highly regulated. The second dimension describes the role of government in health-care provision. At one end of the scale its role is limited to purchasing health-care services from private providers and at the other end of the scale its role is to deliver health-care services directly (provider of health services role). It should be noted that no OECD country's health system can be entirely categorised in only one of the four

1. HTA providers may also envisage use of their products by consumers, but there is little information to indicate whether this occurs. In the view of the authors, HTA is probably relatively inaccessible to most consumers. Whether or not this is appropriate is a separate question which is not addressed here.

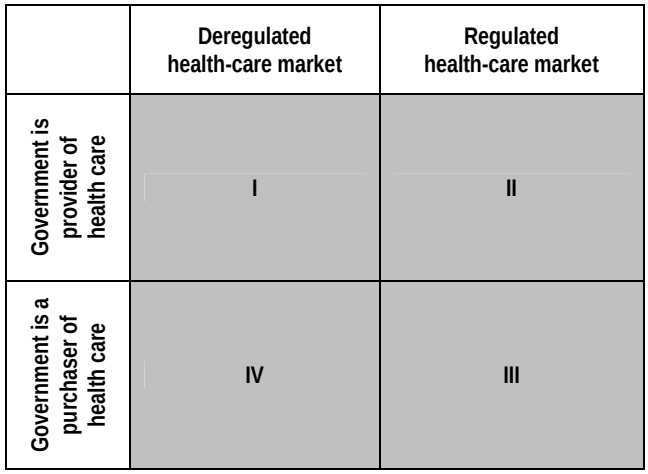
quadrants in Figure 5.1. All OECD member countries, for example, regulate some aspects of their health-care market and leave other sectors less regulated. Similarly, most OECD member countries have some role in the direct provision of health for some sections of the health-care market (or population) and function as purchaser of services in other parts.

The location, along these two dimensions a particular section of a country's health-care system, can influence the level and type of participation of government, private organisations, and other civil society groups in the provision and governance of health-care. Accordingly, the needs for (and uses of) HTA will also vary.

Using Figure 5.1 as a model to describe the health system environment, it can be argued that the use of HTA evidence in decision making will have to take on different forms for different actors in the health-care system, depending on which quadrant the health-care sector is in. What implications does the variation in countries' health-care systems have on the way the different stakeholders in a system use HTA for decision making?

One of the implications, in practical terms, is that many OECD countries have developed HTA agencies, but the ownership of the agencies (public or private), as well as the way that they interact with providers and purchasers of health services, vary in substantial ways.

Figure 5.1. **Health system environment for HTA**



The level of deregulation may play an important part in the market for HTA. In the context of Latin America, for example, there are many more actors involved in decision making than in most OECD countries, and the process is complicated by multiple interests. HTA activities thus play into a picture of multiple, sometimes competing interests and can be regarded with a degree of scepticism. In other health-care sectors HTA might be viewed in the context of a market for the production of evidence, and users (who could see value in such information) might be willing to purchase such information.

Under Australian federal arrangements, the government is a purchaser of health-care for privately provided medical services and pharmaceuticals by way of reimbursements to patients. Strict controls, including consideration of HTA, are applied over what services and pharmaceuticals are or are not eligible for public funding. But this control extends only to certain kinds of health services and products and therefore the scope of matters

that may fall within the purview of central decision making is limited[2]. Central decision makers exercise little control over the specific mix of services provided or locations from where they are provided[3].

In England and Wales, under the National Health Service (NHS), funding is provided to Health Trusts which are responsible for the specific mix of health services provided. Trusts are required, by direction of the secretary of state for health, to make resources available for technologies that central NICE guidance says should be provided within three months of the publication of the guidance. Investment in technology that has not been subject to guidance is a matter for the trusts; but conversely guidance may, as a matter of principle, be provided on any technology in use in the NHS.

Assuming that the objective of HTA is to inform decision making, in line with the functions of HTA as defined by INAHTA, the question is how does one ensure that those functions are considered in decision making and, at the same time, adapted to the particular features of a country's health-care system?

Integrating HTA in the health-care system: experiences from three OECD countries

The Canadian experience

In Canada, 71% of total health expenditure is publicly funded. Health-care provision and purchasing is primarily in the domain of Canada's provinces. HTA capacity development in Canada has been driven by this jurisdictional responsibility.

Canada has a significant history in the production and use of HTA in health-care decision making at both the federal and provincial levels. The first provincial HTA agency in Canada was established in 1988 in Quebec – the *Conseil d'Evaluation des Technologies de la Santé du Québec*, which was replaced by the *Agence d'Evaluation des Technologies et des Modes d'Intervention en Santé* (AETMIS) in 2000. A national agency, the Canadian Co-ordinating Office for Health Technology Assessment (CCOHTA) was established in 1989, followed in 1990 by the establishment of the British Columbia Office of Health Technology Assessment (BCOHTA) and the Health Technology Assessment Unit of the Alberta Heritage Foundation for Medical Research (AHFMR) in 1996. The Ontario Health Technology Advisory Committee (OHTAC) was more recently established in 2003. Health services utilisation agencies that are closely linked to their respective provincial governments have also been created in Manitoba, Ontario and Saskatchewan and undertake some HTA activities. At the national level, CCOHTA's mandate is to co-ordinate all HTA activities across the different jurisdictions and to reduce duplication by other national or provincial/territorial organisations, while also producing assessments and undertaking nationally directed HTA activities[4].

2. For example, it excludes medical services and technologies provided in public hospitals that operate within the policy and decision-making framework of state and territory health authorities.

3. Such controls can be applied but are used extremely sparingly, for example in number of funded magnetic resonance imaging (MRI) and positron emission tomography (PET) units, and in geographic rules about numbers and locations of private pharmacies and pathology sample collection centres).

4. The CCOHTA work programme is approved by a board which includes representatives from each of the provincial health ministries. Suggestions for assessment topics by CCOHTA are sought from external stakeholders, including a web-based topic submission form.

The literature on HTA shows that there is only limited evidence on the use and impact of HTA in decision making[5]. A notable exception is a recent Canadian report on users' views of HTA (Lehoux *et al.*, 2003). This report sets out to determine whether the processes through which Canadian HTA agencies and broader health services research (HSR) agencies[6] report and disseminate their findings are consistent with the expectations and constraints of the various stakeholder groups. The four key HTA user groups were identified as: *a)* health-care provider associations; *b)* government and administration officials; *c)* patient associations; and *d)* the pharmaceutical and biomedical equipment industry.

The report found that the concept of HTA was widely accepted across the four groups, although only about half of the administrators and a smaller proportion of providers were familiar with the work of specific agencies. Patient groups exhibited the lowest level of awareness, with some HTA agencies registering awareness as low as 10%. The survey also found that HTA agencies had established a strong scientific reputation but HTA was by no means the only source of evidence that respondents were accessing.

Messages disseminated by the agencies were perceived as being more compatible with the informational needs of planners and managers than with those of clinicians, patients and the public, even though respondents felt that the agencies understood clinical practices slightly better than administrative practices.

In terms of HTA dissemination activities, the majority of respondents said they received reports on a regular basis from three of the six HTA agencies in this study. However, a vast majority of respondents expressed the desire to systematically obtain such reports. Importantly, user preferences for dissemination varied within and across HTA user groups, indicating that agencies may have to rely on using several methods simultaneously, knowing that each method by itself remains an imperfect instrument.

The survey results show that stakeholders are very willing to collaborate with the HTA agencies. Collaboration could be improved by:

- Consulting administrators and providers to help them tailor assessments to priority needs and concerns.
- Establishing dissemination strategies in collaboration with providers, administrators and patient associations.
- Enhancing interactions between HTA agencies and industry by implementing formalised procedures for obtaining their input and facilitating access to data, and by ensuring that the processes of data analysis and the development of conclusions are fully transparent and scientifically rigorous.

Overall, this study indicated that users of HTA reports were generally satisfied with HTA products.

5. More attention is being paid to this subject at both national and international levels. For example, INAHTA is currently undertaking an HTA impact initiative to review the impact of assessment activities on policy and in the UK, NICE is publishing an assessment of the impact of its guidance on the utilisation, in England and Wales, of the health technologies which it reviewed between 2000 and 2003.

6. The four HTA agencies that were part of this report were CCOHTA (national), AHFMR_HTA (Alberta), AETMIS (Québec) and BCOHTA (British Columbia). The two HSR agencies involved were ICES (Ontario) and HSURC (Saskatchewan).

Canada has been increasing HTA activity. In October 2002, the final report on the state of the health-care system in Canada, "*The Health of Canadians – the Federal Role*" (Standing Senate Committee on Social Affairs, Science and Technology, 2002) was released. This report recommended that the Canadian Federal Government increase the funding it provides for assessing health technology.

And in November 2002 the Romanow (2002) report "*Building on Values: The Future of Health Care in Canada*" highlighted the growing importance of carefully assessing health technology and identified HTA as the driving force to encourage the adoption and implementation of appropriate technologies. The report identified the following obstacles which are impeding technology assessments and the corresponding evidence-based utilisation of health technologies in Canada:

- Inability to effectively identify and set priorities for assessing emerging health technologies, as evidenced by the lack of an early warning system to identify health technologies under development.
- Limited capacity to conduct HTA, resulting in a limited range of technologies being assessed.
- HTAs are not sufficiently comprehensive, either because they fail to fully consider the social, legal and ethical implications of the use of health technologies, or because they fail to provide sufficiently detailed economic evaluations.
- Inadequate primary research data study the relationship between health technologies and overall improvements in health outcomes to support the HTA process.

The Commission recommended that technology assessment in Canada be streamlined to increase its effectiveness, efficiency, and range of topics; and its use in guiding decisions be enhanced. It also suggested collaboration of federal and provincial governments in the development, co-ordination and implementation of a health technology strategy to guide more efficient financing, management and utilisation of technologies within the Canadian health system.

These reports set the stage for the decision, in February 2003, by Canada's federal and provincial first ministers to request that ministers of health develop a comprehensive health technology assessment strategy that would consider the impact of technologies and maximize their effectiveness. To increase the capacity and utility of health technology assessments and to support the development and implementation of the strategy, the Government of Canada provided CAD 45 million over five years to the CCOHTA. These funds are expected to build upon and strengthen existing capacity across the country.

This strategy is currently being developed by a task group with representatives from the federal and provincial governments and CCOHTA. In order to ensure that the strategy is representative of all Canadians, the development process will be consultative, involving all sectors of the health-care system. A completed strategy is expected by late 2004.

The situation in Canada demonstrates that national efforts are underway to recognise and promote health technology assessment as a valuable and necessary tool in health decisions, and that investments in co-ordination and collaboration within countries are essential to ensure more efficient production of HTA as well as its use in decision making.

The Mexican experience

Mexico has participated in international discussions on HTA for a considerable time, but has only recently established institutional support for HTA domestically.

Three important aspects should be considered in assessing the use of HTA information in decision making in Mexico. First, it is important to recognise the complex nature of the Mexican health-care system, and in particular the participation of multiple public institutions and a diverse and autonomous private sector. Secondly, Mexico has well developed scientific organisations that can support HTA efforts, mainly in public research institutions and universities. Finally, Mexico has experience in conducting research on technology assessment, although most of these studies may not been seen as HTA in the formal sense.

The challenge in Mexico has been to create co-ordinating mechanisms, so that institutions can interact in an organised way to drive HTA use in decision making. Developing a national health technology strategy is seen as an integral way of meeting this challenge. In the last three years, there have been significant efforts to face these challenges.

In the private sector a new unit has been established at the Mexican Foundation for Health in order to help co-ordinate the firms that produce health-care technology and the health-care delivery organisations in the country. This unit is created to facilitate the link between the health technology developers (universities), the industries that produce the technology (*e.g.* drug, devices and equipment industry), and the health-care delivery organisations in both the public and private sector. The activities of this unit include HTA, but are more concentrated on the development of innovations and markets for health-care technology. It has a broad mandate, including facilitating appropriate use of technology and the development of incentives for innovation in Mexico.

In the public sector, two initiatives were launched recently. The first one, under the auspices of the Ministry of Health, has created a new body - The Center for Technological Excellence, officially launched in August 2003. Its focus is directed mainly at developing national policies for HTA. The second is the Health Technology Assessment and Management Program that was created at the Mexican Institute of Social Security-IMSS[7].

The IMSS and the Ministry of Health programmes interact through a committee that was created at the initiative of the Ministry of Health to co-ordinate the country's HTA activities.

The development of these two separate initiatives is a consequence of the autonomy of the two institutions (IMSS and the Ministry of Health) combined with the separate roles that each institution has in the Mexican health-care system. That is, the IMSS delivers health-care services directly, while the Ministry of Health is in charge of the

7. The main public health care delivery institution in the country, with coverage of almost 50 million Mexicans.

regulation on the health-care system as a whole. In this situation, the IMSS programme will fulfil its organisational goals (*i.e.* conduct HTA and use it in the delivery of health-care services), but respect the normative leadership that will follow from the Ministry of Health initiative (Durán-Arenas, 2002). These two initiatives are thus complementary.

The IMSS programme's focus is on activities at all three levels of health-care delivery and decision making:

- *At the policy level*. Develop policies to incorporate new medical equipment, including drugs and procedures, that optimise service capacity and, at the same time, keeps the institution at the technological frontier in health care.
- *At the health-care facility level*. Develop information systems and management tools to enhance the capacities of health-care unit executives to manage technology in an effective and efficient way.
- *At the clinical level*. Develop clinical guidelines.

Instances where the programme has already had an impact on decision making are:

- The acquisition and placement of medical equipment based on a registry of the functional status of relevant medical equipment, enabling the rationalised allocation of new equipment based on the needs detected in the registry.
- The introduction of radio-surgery, assessed on the health-care facility's treatment capacity, the number of cases of brain tumours detected within the IMSS population per year, and the optimal geographic location of the equipment to offer better access for patients (Durán-Arenas, 2002).

The use of HTA in health-care decision making is considered a very important issue in Mexico. Work on the effective integration and co-ordination of HTA in decision making has only just commenced but has already seen some success. The challenge ahead for Mexico is for HTA to reach a wider audience of decision makers.

The Australian experience

Like Canada, Australia is a federation and has some aspects of its health-care services delivered through states (provinces in Canada). However, HTA in Australia has mainly been driven by, and has had its greatest impacts on, federal health services arrangements. Australia has national reimbursement schemes for private medical services (Medicare benefits) and privately purchased pharmaceuticals (the Pharmaceutical Benefits Scheme, PBS). States, however, undertake direct service provision for public hospital patients. These are jointly funded by federal and state governments, but are administered by states and are free to patients. In 2000, public expenditure accounted for 69% of total health expenditure in Australia (OECD, 2003).

The production and use of HTA in Australia, at least insofar as decision makers at the federal level are concerned, have been introduced over time to meet the specific needs of particular programmes. That is, HTA is not undertaken by agencies or organisations established or funded for the purpose, but is requested, administered and appraised under the aegis of different parts of the federal health administration (Mitchell, 2002). Examples of the two most formalised arrangements follow.

Pharmaceutical Benefits Advisory Committee

It has been a legislative requirement since 1987 that all decisions for listing on the PBS be considered for comparative effectiveness. Cost-effectiveness evaluation has been a requirement since 1991, and is conducted by the sponsor of the application (usually the manufacturer). Appraisals are conducted by the Pharmaceutical Benefits Advisory Committee (PBAC) and typically are initiated by external applications to the Committee (Mitchell, 2002).

PBAC, as its name suggests, is a committee not an organisation. Secretariat and administrative support to the Committee is provided from within that part of the Department of Health and Ageing (DHA) responsible for administration of the PBS. Technical HTA support used to be provided also from within DHA, but more recently has been obtained under contract to meet the workflow.

Decisions on listing are made by the Minister for Health and Ageing or, if the financial implications are over a certain threshold, by the Australian Government Cabinet. A pharmaceutical can not be listed on the PBS without recommendation by PBAC.

Medical Services Advisory Committee

The Medical Services Advisory Committee (MSAC) was established (administratively, not by legislation) in 1998 with a similar purpose and mode of operation to PBAC but with the aim of supporting decisions for listing of medical services under Medicare benefits arrangements. As with PBAC, MSAC is an appraisals committee rather than an organisation and has administrative support from the DHA. HTA work is performed on request and under contract to DHA. The majority of MSAC HTAs/appraisals arise from external applications (mainly from the medical devices industry). However the Minister, DHA and state health authorities may also refer matters for consideration.

The establishment of HTA as a prerequisite for reimbursement coverage decisions and the integration of HTA and administrative processes have resulted in highly focused and relevant HTA activities. Relevance is enhanced in that while HTA is generally produced by external organisations, members of both PBAC and MSAC are very involved in the development of particular reviews[8]. The focus, though, is of course limited in terms of the totality of clinical practice. A challenge, as noted by Mitchell, arises in terms of maintaining consistency between different HTA activities (Mitchell, 2002).

In general, the concept of HTA is accepted and supported by stakeholders though, unsurprisingly, given the close link with funding, there are concerns about processes. However, these processes are being revised in order to ensure transparency of decisions to stakeholders.

8. In the case of MSAC, each review is undertaken by an external expert organisation with the guidance and assistance of an Advisory Panel (AP), which is a group of clinical, consumer and other experts appointed for the duration of the review. APs include and are chaired by MSAC members.

Use of HTA in other areas of health decision making is developing. Prior to MSAC there were other committees[9] which conducted limited HTAs for both federal and state health administrations on "high-profile" health technology issues[10]. In general, clinical practice guidelines have been developed in a more ad-hoc manner under the aegis of the Australian National Health and Medical Research Council, though recently a National Institute of Clinical Services has been established to undertake this in a more considered way.

A view on the future of utilising HTA and the decision-making process

The case study countries examined in this chapter offer a range of experiences in the production and use of HTA against the backdrop of three very different health-care systems. These countries present useful insights into production and use of HTA.

In Canada, there is widespread experience in producing and accepting HTA. However, a federal system such as Canada's, with shared responsibilities for the delivery of health-care and HTA, faces considerable challenges in the effective and efficient delivery of HTA. As such, recent efforts have focused on generating wider use of HTA in decision making in all parts of the health-care system, as well as more systematic co-ordination and collaboration in HTA.

In Mexico, recent efforts have focused on establishing institutional support aimed at generating wider acceptance of HTA at all levels of health-care decision making. These efforts, while only recent, are already delivering evidence of more informed decision making, but concerted action is required to broaden the use of HTA in the health system overall.

In Australia, the use of HTA is secured at the federal level through two health reimbursements programmes for medical services and pharmaceutical products. Recent initiatives have focused on broadening HTA use in decision making outside of these two programmes by developing clinical practice guidelines, and reforming decision-making processes to improve transparency and stakeholder acceptance.

Despite the many complexities of the individual health-care systems, each of the three countries reviewed in this chapter have made considerable headway in encouraging the use of HTA in decision making. However, even in countries with more extensive HTA experience, there is a need to create appropriate structures and mechanisms that can help consolidate HTA efforts. In Canada, Mexico and Australia there are important initiatives underway to establish or improve those mechanisms, and to develop more efficient processes to assure the proper use of HTA in health system decision making.

The suggested ways forward reflect a trend towards more inclusive HTA. This may include close collaboration between HTA producers and industry to develop more transparent and scientifically rigorous processes. It may also involve developing HTA dissemination strategies in collaboration with providers, administrators and patient associations.

9. The National Health Technology Advisory Panel and later the Australian Health Technology Advisory Committee. Note that requests for HTAs from states came via the Australian Health Ministers' Advisory Committee; that is, from states collectively reflecting joint priorities, and not from individual states.

10. A number of state health authorities have a technology advisory unit which may conduct limited HTA activities. For example, the Southern Health Network in the State of Victoria funds an HTA unit at Monash University, Melbourne.

The experiences also point towards the importance that these three countries place on HTA. As described by INAHTA, HTAs have the capacity to contribute much needed evidence to decision making. All three countries are putting considerable efforts into developing a health system that incorporates HTA and evidence-based medicine, thereby enhancing the use of health-care interventions that are effective and efficient. The country initiatives outlined in this chapter are all very different but the common factor is that the concept of HTA has been tailored to the institutional and organisational frameworks of individual health-care systems.

The effective and appropriate use of HTA requires strong support from policy makers. Just as the producers of HTA have a responsibility to deliver high quality and relevant evidence to decision makers, policy makers have a responsibility to develop the capacity of decision makers and health-care institutions to be receptive to HTA

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Chapter 6

HEALTH-CARE EVALUATION: ISSUES IN TRANSFERABILITY

Corey Magnell, Larry Brown, Alan Moskowitz and Annetine Gelijns
International Centre for Heath Outcomes and Innovation Research (INCHOIR),
Columbia University

This chapter examines the challenges of evidence-based decision making within health-care systems and identifies the potential pitfalls that decision makers face in transferring results from economic evaluations from one setting to another. It sets out three possible areas for policy consideration that may help deal with these pitfalls, including an agenda for improving the dialogue between the analytical enterprise and policy communities.

Introduction

Policy makers and health-care professionals alike are under increasing pressure to understand and manage the health and economic impact of technological change in medicine. Utilising new technologies appropriately and keeping their costs in check, however, is a formidable task. In few fields of public policy are the uses and costs of services so powerfully driven by technological innovation as they are in health care, and that technological innovation never ceases.

As evidenced by the OECD survey results presented in this report, the growing awareness of the causes and consequences of uncertainty in medical practice has spurred the elaboration and implementation of analytic techniques that use hard evidence to tell practitioners, payers, and policy makers which drugs, devices, and procedures work well and which should be modified or abandoned. This portfolio of evidence-based analytical techniques - randomised controlled trials, cost-effectiveness studies, and more - gain legitimacy from the same claims to "hard" scientific validity that medicine itself asserts, but, as with medical science itself, the reception and application of analytic findings varies considerably among and even within nations. Cultural proclivities, supply patterns, specialist organisations, payment methods, medical education, and consumer attitudes all shape the assimilation of "evidence" as surely and opaquely as they shape the divergent practice patterns which evidence is expected to rationalise; so too do national images of equity, the scope of bureaucratic autonomy, the prowess of decision making to participation by interest groups, and the myriad other forces that shape societies' health policies.

Although cross-national differences in the confrontation between analytic counsel and medical practice have yet to attract much deep exploration, variations are nevertheless evident. Evidently, analytical insights are not neutral pieces of knowledge to be transferred like so many interchangeable parts among (or even within) societies. As Clifford Geertz remarked, "it matters where things happen" (Geertz, 1983) and a fair amount of local knowledge is needed to gauge if and how particular evidence-based tools will work, and where and when.

This chapter briefly considers the challenges of evidence-based decision making within health-care systems founded on different principles, and the prospects for the productive transfer of evidence-based studies among nations. It then sketches an agenda for improving the dialogue between the analytical enterprise and policy communities.

Evidence-based decision-making processes

As argued throughout this report, better information on the effectiveness and cost-effectiveness of clinical interventions can steer clinicians and policy makers toward more appropriate uses of technology. Consequently, industrialised nations have increased their investment in clinical evaluative research to provide a stronger empirical foundation for decisions about technological change. What then are the decision tools available to manage technological change?

OECD countries use regulatory tools and public planning to provide, limit, and distribute the supply of medical technology. Industrialised nations regulate the introduction of new drugs and devices. In addition to these pre-marketing controls, these countries may require hospitals to obtain public licenses for expensive devices and procedures, such as lithotripters, open heart surgery centres, nuclear medicine imaging, and genetic screening. These planning mechanisms are directed chiefly at high-tech interventions, whereas financial incentives may shape the use of all health-care technologies.

On the demand side, Beveridge-type, or single-payer systems of the kind found in the United Kingdom operate with a control structure consisting of global and regional budgets. Bismarckian systems, such as those in Germany and France, employ coverage and reimbursement decisions made by insurers to shape the use of technology in clinical practice. Specialty societies, government agencies, and insurers, meanwhile, issue guidelines regarding appropriateness of use.

Budgetary caps at the national, regional, or hospital level explicitly force choices among technologies and tend to dampen the overall rate of diffusion. But budget-driven constraints do not necessarily trigger the selection of the most effective or cost-effective products. A prerequisite for the rational allocation of resources within a fixed regime is information on the net effectiveness and costs of medical interventions, and this detailed information is often not available.

Increasingly, health-care systems have invested in strengthening the analytical enterprise. In the United Kingdom, for example, the National Institute for Clinical Excellence, founded in 1999, makes central recommendations to the National Health Service (NHS) about the utility of particular treatments. Bismarckian systems make detailed coverage and reimbursement decisions that rigidly define where society's obligations end and individual responsibility begins. Over time, coverage decisions have become more informed by evidence not only of effectiveness but also of cost-effectiveness. In Australia and certain provinces of Canada, for example, sponsors must provide cost-effectiveness data to obtain approval for public reimbursement of a new

drug. As a result of this demand for cost data, there is ever-expanding literature on the cost-effectiveness ratios of new and commonly used technologies.

Over time, both these supply-side (*e.g.* pre-marketing regulation of drugs and devices) and demand-side (*e.g.* coverage and reimbursement decisions) policy mechanisms have come to rely increasingly on empirical evidence. Better knowledge about the risks, benefits, and costs of technologies have reduced the uncertainty involved in these decision-making processes, and have resulted in more informed decision making. At the same time, there are several important constraints to relying solely on empirical evidence.

Constraints of current evidence-based practices

Lag between evaluation and practice

The rapid pace of technological change, and the expansions of use that evolve in practice, often cause rigorous evaluation to lag behind current practices. Coronary bypass surgery, for example is now used as treatment for chronic congestive heart failure. Although this practice has gone on for some time, only recently has a randomised trial been initiated to address the value of revascularisation surgery in treating heart failure (Surgical Treatment for Ischemic Heart Failure (STICH), see Joyce *et al.*, 2003). In the field of telemedicine, efforts to evaluate the cost-effectiveness of the technology are quickly out of date because of the rapid advances in the quality, applications and costs of information and communication technologies (Ohinmaa *et al.*, 1999).

Importance of qualitative factors

Even when the knowledge base concerning a particular clinical intervention is robust and current, clinical and policy decisions require the integration of empirical evidence with qualitative factors. Consider pre-marketing regulatory decisions, which rely increasingly on quantitative data provided by randomised trials to determine the efficacy and safety of a new drug or device. However, societies determine the acceptability of trade-offs in risks and benefits provided by a new technology based on value judgments, which are ultimately qualitative in nature.

Shortly after its approval, flosequinan, an oral inotropic heart failure drug which improved cardiac function and quality of life, was found to reduce survival. As a result, the drug was withdrawn from the US market. Yet, when this risk benefit trade-off was presented to patients with heart failure, a disease which compromises both quality of life and survival, 40% stated that they would accept a slightly higher risk of death ($\geq$5%) to achieve a better quality of life (equivalent to a 5-point change on the Minnesota Living with Heart Failure Scale) (Rector, 1995). This exemplifies how pivotal value judgments are in health policy decision making. The implementation of such judgments lends itself to international variations in policy.

Dideoxyinosine (ddI), a retroviral agent, and RU-486, a contraceptive agent, were two innovations where social value judgments played a major role in the national regulatory approval process. Political pressure from activist groups in the United States was a major impetus for the FDA's enactment of a rapid approval process, which paved the way for the use of ddI for patients with AIDS. In contrast, RU-486 became a captive of the abortion debate in the United States and saw its approval delayed. These cases suggest a pervasive role for qualitative concerns, like population attitudes towards risk, and societal values in our decision making.

The use of cost-effectiveness and cost-utility data in coverage and reimbursement decisions generates similar types of questions. Should these CE ratios be used, as some health economists argue, as strict thresholds? Such a practice may be problematic, because it does not leave room for important qualitative aspects to play a role in decision making. These ratios, for example, do not capture such important factors as the role of equity and distributive justice in determining the place of new technologies in financially burdened health-care systems.

The use of quality-adjusted life years (QALYs) as a measure of effectiveness may encourage political divisiveness by juxtaposing the interests of the young and the old, who have inherently less capacity to benefit from interventions because of their shorter life expectancy. Such issues must be addressed pre-emptively, much as the Dutch did in the early 1990s when, in their report on *Making Choices in Health Care*, they suggested using a social utility function in addition to cost-effectiveness ratios in forming new health-care policies in order to ensure equity in the allocation of funding to the elderly.

Limited resources

Finally, the resources available for conducting clinical evaluative research are finite. Conducting clinical trials is a costly exercise and priorities must be set as to which of the many new and expanded technologies in existence today requires evaluation. In addition to monetary resources, patient resources may be constrained. Although science is built on the concept of confirmation, trials designed to substantiate prior results may present an ethical dilemma in many instances, particularly in the case of life threatening diseases. For example, a recently published trial comparing implantation of left ventricular assist devices to medical management in the treatment of end-stage heart failure showed a major survival benefit of using the device (REMATCH, see Oz *et al.*, 2003). The effectiveness and adverse event profile of this technology, and the outcome of the control population, are likely to vary substantially depending on the expertise of the treating institutions, which argues for repeating the trial in different settings. However, duplicating such a trial may be unethical because of the new evidence that one therapy is better than the other. These and monetary restrictions mean that the results of clinical trials and cost-effectiveness analyses done in one country or setting need to be transferred to another setting without new randomised trials being carried out.

Problems in transferability

Matching research techniques with policy-making needs is a bewildering proposition for all involved. There are countless sources of variation to consider when adapting technological assessment to a particular health-care setting which, if not addressed, might lead to the inefficient implementation of a new technology. Among the more transparent differences among settings are the unit costs, the prevalence and severity of a disease in the population, as a result of variations in such factors as ethnic makeup, lifestyles and age distribution of a region. Such variables may be addressed by substituting local prices and epidemiological data. Other factors which have an important effect on the transferability of technology assessment, however, are far less amenable to straightforward calculations. Local communities differ greatly in the relative efficiency of their health-care systems, leading to potentially vast differences in the effect of a new technology. Differences in incentives and payment systems may affect the diffusion of technologies.

Table 6.1. **Comparison of four cost-effectiveness analyses on screening for abdominal aortic aneurysms**

Study	Type	Level of detail in costing: screening	Level of detail in costing: surgery	Endpoint	Timeframe	Cost/life year (USD)
Multicentre Aneurysm Screening Study Group (2002) United Kingdom	Prospective 67 800 men	Clerical staff time, postage and stationery, cost of obtaining patient details, office space and equipment, clinic staff time, staff travel costs, disposables, maintenance of equipment	Time in intensive care, high dependency units, general surgical wards, OR time (including staff time), cost of graft used, fixed cost for consumables for each center, drug use based on sub-sample of 60 patients, blood product use and handling charges, hospital-specific laboratory charges	Abdominal aortic aneurysm (AAA)-related deaths	Four years	47 607
Lindholt (2002) Denmark	Prospective 12 700 men	Unclear: costs registered prospectively for first year of trial, indirect costs calculated from questionnaires of sample of patients	Calculated from random sample of 100 operations performed at single site, components unclear	AAA-related hospital deaths	Seven years	1 185
Lee (2002) United States	Markov decision analysis model Hypothetical cohort of 70-year old men	Based on Medicare reimbursement rates	From hospital cost-accounting system, details in referenced publication	Costs	Death of all patients	11 215 per QALY (based on estimation of effect on QoL by researchers)
Pentikäinen (2000) Finland	Monte Carlo simulation Data from own empirical studies	Direct costs based on hospital screening data, components unclear	Based on small sample of operations: cost of operation, intensive care, hospital stay, medications, blood products, laboratory and radiography, equipment overheads	Not included	Death of all patients	9 431

Each health-care system has its own unique cache of resources, leading to differences in opportunity costs among settings when a new technology is considered. Likewise, each local setting has its own standards of care and accepted practices, which might not be directly comparable to the baseline technology used in a cost utility analysis. Social values and preferences of patients are known to vary substantially among countries. Given the large numbers of variables, many intangible, which may affect a study's validity in local settings, policy makers are faced with the difficult task of how best to interpret evidence, at the risk of wasting resources.

Obviously it is not possible for investigators evaluating a new technology to tailor studies to every community which might adopt it in the future. The current state of cost-effectiveness literature, however, lacks consistency in how data are collected, analysed, and reported, making transferability of results cumbersome. An analysis by Späth *et al.* (1999) of cost-effectiveness analyses of adjuvant chemotherapy in breast cancer revealed a pervasive lack of standards in reporting results: of the 26 studies found in the literature, only six met criteria for external validity and none was directly transferable to the French health-care system in particular. The most common obstacles to transferability were a lack of detail in reporting health-care resources and prices used and a lack of specificity in defining the setting in which the study took place. A study by Stone *et al.* (2000) showed striking differences in the methods used to estimate costs in a cohort of similar studies. Few of these studies followed accepted guidelines in cost-effectiveness analysis.

A review of four recent cost-effectiveness analyses of screening for abdominal aortic aneurysms demonstrates the diversity of current research techniques and the problem with accepting the results of even published studies at face value (Table 6.1). Each study analyses the cost-effectiveness of targeted screening for abdominal aortic aneurysms using abdominal ultrasound, taking into account both the cost of screening and associated treatment costs.

While each addressed the same analytical problem, there was great variation in their reported results. Cost-effectiveness ranged from USD 1 185/life year saved to USD 47 607. The source of this variation is difficult to discern due to the lack of detail in describing costing methodology and results in some of the published reports. The studies also differed greatly in their choices of endpoints and timeframe, which could also contribute to this diversity of results. Two studies measured aneurysm-related deaths while the other two computed all-cause mortality in their cohorts. The timeframe for measuring effectiveness ranged from four years to the life of the cohort. Such diversity in methods and lack of detail in reporting precludes the easy transfer of cost-effectiveness results from one health-care setting to the next.

Similarly, there is no clear consensus among clinical researchers regarding which patient populations to study. Russell and Sisk (2000) looked at how age differences had been taken into account in 36 cost-effectiveness studies. They reported that most studies only mentioned age tangentially - only 36% of the studies examined differences in the effectiveness of the technology by age and likewise failed to stratify risk and costs by age. The authors also note that while age is often used as a rough indicator of risk, it may mask groups of individuals with different risk and benefit profiles as well as different costs. Age, then, may not be a useful demographic category in cost-effectiveness analysis in the absence of social and physiological qualifiers.

The conscientious decision maker, who wisely consults the economic literature before making a decision, is not served well by the lack of transferability between settings. It is perhaps not surprising that several reviews have found that economic evaluations are

often not used by decision makers, and that a key reason for this is the lack of transferability of study results (Hoffman *et al.*, 2002; Drummond and Pang, 2001).

Accepted practices in clinical research are often at odds with the practical needs of local policy makers in other arenas as well. Clinical research is first and foremost concerned with efficacy and safety. Trials are designed with this in mind and are often ended early when both of these criteria are demonstrated in a statistically significant way. While this practice brings effective technologies to market quickly, it is not conducive to calculating long-term costs of an intervention. Local communities must thus invest in these technologies with only short-term evidence of their cost-effectiveness at their disposal.

Rarely does technological assessment take "other determinants of health," such as education and social support structures, into account. This is usually inherent in the study design - clinical science demands that any experimental group be matched with an identical control group. Study groups are thus usually randomised with respect to race, gender, social status, etc. While this may minimise the effects these factors might have on study results, the practice precludes policy makers from determining which subgroups might benefit most from an intervention, as it emphasises the effect on an "average" population. Although lesser-advantaged populations can often benefit the most from health-care spending, current standards in technological research fail to give decision makers the arsenal of data needed to enact policies that deliver more equitable and efficient health outcomes (Birch, 1997).

Health economists typically argue that cost-effectiveness analyses need to be conducted from the societal perspective or from that of the health-care system. Such perspectives allow an understanding of how investments in one technology category (*e.g.* drugs) may lead to savings in other parts of the health-care system (*e.g.* hospital use) or to savings accrued in other years. Decision makers, however, are often confronted with "silo" budgeting systems. For example, in many countries drug budgets are separate from hospital budgets and there is little opportunity to transfer resources across budgets to facilitate gains in patient health and overall efficiency. Moreover, if improvements in patient health care require substantial short-term investments to obtain long-term benefits, budget realities in a given year may not allow for such increases.

Options for addressing the issues

Notwithstanding the substantial gains from advances in evidence-based decision making, several shortcomings have been identified in the current status quo, such as the significant delays in definitive evaluation of technologies, failure to integrate qualitative factors into analysis, pervasive lack of resources for evaluative enterprises, and problems with the transferability of the results of evaluative research. Three areas for potential improvement are discussed here: strengthening the health evaluation activities; adhering to methodological and data reporting standards, and strengthening the decision-making processes to integrate qualitative and quantitative factors.

Strengthening the analytical enterprise

Strengthening the analytical enterprise (including clinical evaluative research for health technology) would require a stable and adequate funding base that would support the acquisition of new data and the advancement of the analytical tools. The health sectors of OECD member countries are highly research intensive. Clinical evaluative

research typically falls far behind basic medical research in terms of funding availability. The multinational pharmaceutical and medical device industries spend nearly 20% of gross sales on research and development (R&D). Roughly 30% of these budgets go toward pre-marketing and post-marketing clinical trials. These trials are conducted with the explicit objective of satisfying regulatory, coverage, and reimbursement requirements.

Government agencies, such as the US National Institute of Health, also support the conduct of clinical evaluative research. In 1999, that agency spent 10% (USD 1.47 billion) of its overall budget on health technology evaluations. These trials are important because they have a different focus than private sector trials. In particular, they support evaluations of major clinical procedures and off-label therapies. In the United States, the combined spending of private industry and federal R&D organisations on clinical evaluative research is in the order of 1% of health-care expenditures.

In view of the size of the basic medical research budget, and the uncertainty that exists about the appropriate use and economic consequences of the resulting innovations, one could argue for an increased investment in clinical evaluative research. What should be the size of the increased investment and what role the various stakeholders should take in supporting it is a complicated question. Each sector (industry, public R&D organisations, insurance institutions) generally would prefer that other bodies shoulder more of the burden, but as both the number of evaluations and their complexity and sophistication increase, the need for intersectoral partnerships to pool resources becomes more evident. Over and above such efforts to acquire additional data, there are measures that could be taken to maximise the use of studies that are being conducted.

Strengthening data and methods

Greater efforts among researchers to standardise the conduct and reporting of cost-effectiveness analyses in publications would greatly facilitate the translation of these studies to diverse settings. A number of studies, including the analysis presented here, have identified an important lack of detail throughout the cost-effectiveness literature in reporting the health-care resources utilised and unit costs of an intervention, as well as effectiveness parameters, and sub-groups of patients in whom benefits are seen (Russell, 2000; Birch, 1997).

Adding to this uncertainty, the perspective of published analyses often may not coincide with that of primary decision makers (*e.g.* health insurer, hospital administrator). Although the perspective for all cost-effectiveness analyses should be the societal one, juxtaposing the perspectives of other relevant decision makers would offer important insights into practical barriers to implementation of cost-effectiveness recommendations.

A number of OECD countries, as well as academic centres and health technology assessment agencies, have published guidelines and recommendations for standards in conducting and the reporting of cost-effectiveness analyses. The issue of greater transparency in the reporting of cost-effectiveness analysis is a key consideration in these. Gold *et al.* (1996), for example, recommend that the reporting of cost-effectiveness analysis in journals might better be done within a framework that would include a technical addendum to clarify assumptions and methods used in the analysis, validate the model employed, provide detailed sensitivity analysis (*e.g.* variation in the perspective of the analysis) and disaggregated results.

Strengthening the decision-making processes

Quantitative evidence seldom if ever speaks for itself; that is, it tends not to unequivocally indicate to policy makers what course to take in allocating health-care resources. The decision-making process could be strengthened by including patient groups, clinicians, and the representative public in the appraisal of technologies. The adoption and acceptance of technologies is critically limited by societal values and by the opinions and assumptions, informed or not, of "front-line" practitioners. Particularly in the case of technologies with limited applications in critical or debilitating diseases, patients who may be significantly affected by these decisions should be included in the interpretation of evidence in order to foster humane and efficient policy making. Increased transparency of the decision-making process in addition to the open acknowledgment of ethical concerns and local values can enhance the decision-making process and generate greater legitimacy in the eyes of the public.

The distinction between appraisal (the qualitative judgement and interpretation of analysis) and assessment (an objective analysis) is important. Decision makers require as much objective analysis as possible to help them in their quest to make informed decisions but there is also a need to consider the techniques by which objective analysis can be appraised. One important question is: where should the full breadth of perspectives on new technology come into play? That is, in the sequence from assessment to appraisal to decision making, where should these wider perspectives be incorporated? Can they be delivered within the margins of health technology assessment or is there a need for a separation of the appraisal and assessment roles? The answer will, to a considerable extent, depend on the institutional and organisational features of the health-care system, including the role that HTA plays in that system.

A further question centres on how policy makers can institutionalise the interplay between the appraisal and assessment? In fact, this is often easier said than done. It is unclear what kinds of qualitative methods offer what types of benefits in different decision-making contexts. Qualitative methods are anything but homogenous, ranging as they do across the social sciences (sociology, political science, economics, anthropology, psychology), the humanities (history, philosophy), and such hybrids as bioethics. Deciding how much input one wants, or can usefully harness, from these various fields depends less on their *a priori* merits and claims than on the kinds of questions researchers and policy makers want answered.

Conclusions

OECD countries use a wide range of policy and planning tools to provide, limit, and distribute the supply of medical technology. Increasingly, these tools have come to rely on empirical evidence and OECD countries are investing greater resources into the health evaluations to guide decision making. It is clear that better empirical evidence about the costs and consequences of medical technologies can make policy decisions sharper and better grounded.

However, there are several important challenges in using empirical evidence, including a potential lag between health-care evaluation and technological development, equity and distributive justice considerations, uncertainty over whether evidence can be transferred from setting to setting and an inability to determine which subgroups might benefit most from an intervention. This latter issue will become a bigger challenge as pharmacogenomics-based products come online.

Furthermore, quantitative evidence should never purport to be the sole source of important social judgments. Once clinical and economic evidence have had their due, culture, ethics, psychology and politics will, and should, complicate the equation with their own irreducible claims.

Three possible implications for policy have been put forward in this chapter:

- Strengthen the analytical enterprise through new public and private partnerships that enable the costs of health evaluations to be shared.
- Include wider stakeholders (patients, health-care providers and public representatives) to help bring wider perspectives to the appraisal and decision-making process.
- Develop reporting frameworks which make it easier for decision makers to assess the relevance of economic evaluations to their local setting, and more easily extrapolate the results.

The challenges surrounding these issues have been too little explored, and suggest an important agenda for further policy research.

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Chapter 7

MANAGING DECISION MAKING UNDER UNCERTAINTY: PERSPECTIVES FROM A CENTRAL ADMINISTRATOR

Damian Coburn
Department of Health and Ageing, Australia

Uncertainty is pervasive in health-care decision making and arises when there is insufficient definitive information to make informed choices. This chapter examines the challenges that decision makers have to confront when faced with uncertainty. Its focus is on the administrative contexts of decision making and provides some available approaches for managing uncertainty within that context.

Introduction

Health-care decisions taken by governments or by insurance companies are often made in the context of formulary listing, reimbursement of a new medical technology or investment in a public screening programme. Evidence, including information on whether a new technology presents value for money, plays a key part in aiding decision makers to make informed choices.

However, evidence is not always available to make informed decisions, and even when it is, some uncertainty will remain. Such uncertainty may arise when there are conflicting results from various sources of evidence or when evidence suggests a range of possible outcomes and it is only possible to make a best guess at, for example, the cost-effectiveness of the new technology.

Uncertainty creates problems for decision makers because they are charged with choosing between various scenarios when there is insufficient definitive information on which to base decisions. In health care, the stakes for such decisions are high and may carry both high financial and health risks and rewards. Furthermore, the overarching imperative and responsibility for decision makers is to make decisions, even if on poor quality evidence (Taylor, 2002). To defer consideration of a matter until the perfect evidence is in is, in effect, to decide.

This chapter examines the challenges that decision makers have to confront when faced with uncertainty, and examines available options for managing uncertainty. It focuses on the administrative contexts of decision making and how that affects handling of uncertainty. For the purposes of this chapter, the term "decision maker" is centred on administrative rather than clinical decision makers. Administrative decision makers may include ministers, members of an appraisal committee, government officials and health insurance administrators (such decision makers are sometimes referred to as generating macro and meso rather than micro level decisions).

Health technology decision making – the administrative context

The essential question regarding health technology is: "should we use it?" Health-care providers are typically charged with the decision to recommend whether or not a certain technology should be used at the individual patient level. However, decisions made at the macro and meso levels also influence the answer to this question. These questions tend to focus on:

- Should this technology be paid for/reimbursed (*e.g.* should this new drug be listed on the national formulary)?
- Who should have access to this technology (*e.g.* should free mammography screening be made available to women under the age of 40)?
- Where should the technology be available (*e.g.* is there a need for a new CT scanner in town *x*)?
- Should use of the new technology be encouraged in preference to older technologies?

These questions are answered within the framework of a number of health-care system objectives, including:

- *Promoting access to provision of effective and appropriate health services*. Decision makers are concerned that individuals within their jurisdictions have access to high-quality health services that will improve their health status. Decision makers also aim to restrict access to interventions that are ineffective, harmful, or not deemed to be appropriate for (often public) reimbursement.
- *Promote equity of access to service provision.* Poor health status, and/or access to health services, is sometimes associated with one or more socio-economic or demographic factors, for example, being of low income or being a member of a particular (*e.g.* indigenous) community. Decision makers are generally concerned with promoting equitable access to health services on the basis of clinical need[1].
- *Efficiency of health service provision.* Throughout many OECD countries, health costs are rising in real terms, often at a greater rate than other government programmes. A major objective in most health systems is to promote value for money within the constraints of fiscal policy, while trying to balance this against other health-care objectives.

1. It should be noted that an explicit aim in the establishment of the UK National Institute for Clinical Excellence (NICE) was to address "postcode prescribing" (Dobson, 1999).

Box 7.1. Professor Smith goes decision making

Professor Smith was on her way to a meeting of a national health technology appraisal committee, the first since her appointment. She was feeling a little uncomfortable and even a little out of her depth because, while she had a good grasp of clinical trial methodology and could even follow an economic evaluation, deciding on access to procedures for a whole country was very different to making clinical judgements for her patients, or advising her students.

Young Dr. Jones in her department had been talking just yesterday with enthusiasm about one of the technologies she would be considering today. Yet the technology evaluation reports that had been delivered to her office seemed so inconclusive. There was nothing new in the reports that had not already been published in learned journal articles, but the technology had some alarming numbers in the economic analysis.

This was also a source of discomfort: how does money enter the equation of patient care even when the technology seemed intuitively to be a good idea. Professor Smith shifted uncomfortably in her seat: she imagined she would have to listen to some purist academic epidemiologists and health economists who would want to deny everything to needy patients just because there are not enough randomised controlled trials.

There are also environmental factors that shape how decision makers operate. As alluded to in the story of Professor Smith in Box 1, some of these factors involve preferences or expectations that may constrain, in practical terms, the feasibility of certain decisions. Jan (2003) and Rosen and Gabbay (1999) provide an analyses of how decision and policy making is performed while noting that there is little literature in this area. Jan examines why decision makers do not use economic evaluation and suggests that they are often unwilling to make difficult choices. Rosen and Gabbay also refer to the influences and expectations of external stakeholders. These issues are explored below.

- *Stakeholder interests.* Providers of health products and services have a direct interest in health technology decision making, especially around financing coverage. The views of decision makers and providers of health-care services may sometimes diverge. Decision makers at the macro and meso levels are often concerned with population level impacts, whereas the doctor and patient are concerned with individual health impacts (McDaid, 2003; Maynard and McDaid, 2002). These competing interests can create tensions for decision making given a further set of related factors, namely lack of information and information asymmetry, the "technological imperative", and moral hazard, which are outlined in detail below:
    - *Lack of information and information asymmetry.* Patients use doctors as their agents in delivering advice and services about the possibilities for their health-care, though this is becoming less the rule as consumer awareness is becoming part of the health service landscape (Sanders, 2002). However in circumstances where many or most health interventions have not been subject to systematic and/or evidence-based review, and given the rapid rate of innovation in health-care, in some circumstances health-care providers may also lack knowledge. This may be because in relation to "established" health technologies, the "truth" has yet to be systematically or scientifically investigated; in relation to innovations, it could be because the evidence is not available yet. As Maynard and McDaid (2002) note, and as is well known, health technologies are frequently disseminate prior to evaluation.

  - *The "technological imperative".* As stated by Briones et al. (1999) "health-care professionals are very receptive to new technological developments and often make decisions based on the idea that anything that might benefit my patient in some way must be at least tried and so, financed". In turn, patient expectations may be raised about the possibilities of new innovations after favourable media coverage.
  - *Moral hazard.* Health insurance (both public and private) can increase demand and create economic inefficiencies. As the ultimate consumers often do not face the full costs, the incentive for both patients and health-care providers is to over-utilise health-care services.

- *Difficult choices, social preferences and expectations.* According to Maynard and McDaid (2002) "decision makers will continue to allocate resources inefficiently because such practices are consistent with often rather ill-defined concepts of equity". For example, many decision makers are willing to sacrifice efficiency if the extra costs deliver benefits to more deserving groups in society, such as children, veterans or patients with a high burden of disease. Such practices might be inefficient but consistent with social preferences or equity considerations. The clear articulation of social preferences which can be applied in scientific or economic analyses is complex and likely to be highly controversial (Ham, 1997). In the context of the present environmental analysis, it suffices to note that decision makers are faced with social imperatives which influence decision making.

Thus there is a range of expectations about access to health technologies, which may be more or less well informed by science. In addition, the decision makers who have to make choices in this complex environment of multiple health system objectives, social preferences and rising stakeholder expectations may not be experts in the technical methodologies of evaluating health technologies.

Managing uncertainty

Uncertainty is considered here as a source of risk that decision makers must manage. Evaluators of clinical research are at risk of making two kinds of errors. They may conclude a treatment is effective when it is not, or conclude that a treatment is ineffective when it is actually effective. Decision makers also risk making these errors (Taylor, 2002). The decision maker risks approving or encouraging access to a technology that may be ineffective, inefficient or even harmful (say, by approving reimbursement); or the decision-maker may impede or deny access to technologies that are beneficial and efficient.

Uncertainty arises when decision makers face a reduced ability to answer the following kinds of questions:

- Which individuals will benefit from the technology?
- By how much and for how long will they benefit?
- How much do these benefits differ from current standard treatment, and what is their cost?

Uncertainty may arise because key data may not have been subject to any research at all, or may not be amenable to discovery though clinical trials. Box 7.2 provides a scenario (positron emission tomography, PET) relating to the inadequate and absent research that faced decision makers in Australia, and that also illustrates some of the contextual factors discussed above.

There is a substantial literature around uncertainty, especially in the health economics literature. The very great majority of this, however, is directed to health economists rather than to decision makers. However, there is a small, but growing, literature about decision making and uncertainty. This tends to fall into either or both of the following categories:

- Descriptive results of surveys, focus groups and other techniques of finding out what decision makers think of health technology assessment (HTA) and economic evaluations, and what they want to see in them. Recent examples can be found from UK and Canadian experiences, including Drummond *et al.* (2003), Lehoux *et al.* (2003) and AHFMR[2] (2003).
- Prescriptions on how producers of economic evaluations should present information (including information that is uncertain) so that it is more useful for decision makers. For example Drummond *et al.* (2003) and Briggs and Gray (1999) provide useful reviews of technical and methodological issues in handling uncertainty. An interesting related development in this area is the guidelines produced by HTA agencies. Many, if not most, agencies have such guidelines either to provide templates for applicants and/or to define what content HTAs shall have, and how they will be conducted. However, more recently some HTA agencies have developed guidelines to assist decision makers in situations of uncertainty pertaining to emerging technologies. These include Briones *et al.* (1999), Hailey and Harstall (2001), and ASERNIP-S[3] (2002). The ASERNIP-S guidelines are notably aimed at decision makers at the level of a hospital or local health service.

Both categories deliver helpful contributions to the literature. However, what is possibly missing is substantial mutual engagement on HTA methodology and practice between producers of HTA and decision makers - its users.

Prescriptive advice for decision makers in handling uncertainty is a topic for which more rigorous research is needed. The following are possible approaches that might be used to aid decision making in the face of uncertainty[4].

2. Alberta Heritage Foundation for Medical Research (AHFMR).

3. Australian Safety and Efficacy Register of New Interventional Procedures – Surgical (ASERNIP-S).

4. These draw on the ten commandments developed as part of the project on "Analysis of the Scientific and Technical Evaluation of Health Care Interventions in the European Union (ASTEC)" (Maynard, 2002).

Box 7.2. Decision making on positron emission tomography

PET is an imaging modality with a variety of applications but especially in oncology. In oncology applications one of the main uses of PET is in staging; that is, confirming spread of disease to inform treatment planning. The principal claim for PET in oncology is that it can detect disseminated cancers that cannot be seen on other imaging modalities (particularly computed tomography, CT). The value of this appears logical and plausible, however to be useful to patients (and leaving aside cost-effectiveness for the moment) it needs to be demonstrated that:

1. PET has superior accuracy to CT in identifying disseminated disease.
2. As a consequence patient management plans are changed.
3. Changed patient management plans are actually carried out (noting this may mean foregoing treatment).
4. These result in net benefits for patient well-being.

The Australian HTA report on PET found that for a range of clinical uses there is reasonable evidence for 1, some evidence for 2, but little or no evidence for 3 and 4. This has created some controversy (as have HTAs produced in other countries and which have come to similar conclusions). Other factors were that:

- Cancer is a national health priority in Australia.
- There is a strong belief on the part of medical practitioners in the utility of PET.
- At the time of the HTA two PET sites were already funded in Australia.
- PET is very high cost, both to establish and to operate.

Rapid rates of dissemination of imaging diagnostics have been of concern in Australia in the past.

The outcome for PET in Australia was that reimbursement was provided to an increased, but limited number of sites (eight sites; PET and MRI are the only imaging modalities that are subject to limits on the number of federally reimbursed service providers in Australia); funding is contingent on clinical data collection to enable further HTA in three years' time.

i. Develop dialogue and understanding with producers of HTA.

Producers and users of HTA operate in different environments and under different imperatives. Increased mutual understanding is likely to increase the usefulness and relevance of HTA and economic evaluations and create opportunities for the development of more practical guidance on decision making. At the same time, care needs to be taken to ensure that methodologies and the practice of HTA remain sound and impartial.

ii. Try to understand HTA.

Those whose "core business" is technology decision making, and who are in proximity to (or inside) appraisal processes, may benefit from training in HTA methods if the following suggestions are to be of assistance. If users of HTA do not understand it, they are unlikely to be able to ask the right questions. Hence they may get the wrong answers without realising it.

iii. Insist on the underlying data.

Drummond *et al.* (2003) make a number of recommendations as to how economic evaluations can be made more transparent and accessible to decision makers. From the decision maker's perspective, it is critical to understand the limitations of data so as to be able to make judgements about the strength of evidence against policy objectives.

iv. Avoid summaries provided by measures such as ICER[5].

Uncertainty is uncomfortable for decision makers. ICERs (especially of the point estimate variety) offer false comfort; they appear to be a neat summary measure of all the information needed for decision making. Furthermore they seem to package all the information in a way that allows it to be assessed against a shadow price (explicit or otherwise) to readily provide the answer to every technology question. This, however, can be dangerous as it does not allow unpacking of the context of the technology to allow judgements to be made in conjunction with broader objectives. For example, who and how many are affected, and what is their health condition? Undue focus on the point estimate would ignore uncertainty.

v. While being daring in the use of economic evaluation tools, beware of their limitations.

Gafni and Birch (1993, 2003a), and Birch and Gafni (2002) warn against the inappropriate use of cost-effectiveness evaluation in decision making. They argue that the inappropriate use of cost-effectiveness analysis (CEA) is at best inconsistent with policy objectives and in Canada has resulted in adverse consequences: "decision makers can be blamed for consistently failing to recognise that the use of such methodology is a prescription for uncontrolled growth in expenditures" (Gafni and Birch 2003b). More precisely, according to Birch and Gafni, the use of CEA within the framework of a fixed budget is only appropriate when used in conjunction with the concept of opportunity costs. They contend that decision makers have used CEA just to decide whether a technology should be funded within a health system, with little regard for global budget implications.

In a survey of OECD countries, Dickson *et al*. (2003) found that for most OECD survey countries the objective of using CEA in pharmaceutical decision making was to improve value for money, and not necessarily to contain costs. In situations of uncertainty, more information is generally better than less and CEA can be used to obtain a grasp on the extent to which a technology is "value for money", even if that is not a complete or proper use of the metric.

5. ICER: incremental cost-effectiveness ratio.

vi. Don't be limited to "all or nothing" approaches or single benchmarks for evidence.

Decision making is a risk-management problem; and a risk-management approach can be useful in judging the methodological rigour required to assess and appraise the strength and quality of evidence. Factors such as availability of alternative treatments, seriousness and prevalence of disease, likelihood of harm, and potential budget impact, may all bear on the resources put into HTA, the strength of evidence required for effectiveness and the acceptable shadow price for a technology. Risk factors may also influence the acceptability of modelled economic evaluations in regard to extending time horizons of trials or extrapolating final health outcomes from surrogate outcome data.

The controlled and limited diffusion of technologies can be a useful approach to allowing demonstration of the effectiveness and cost-effectiveness of promising technologies. In Australia and Switzerland, for example, promising medical technologies may be given funding coverage on a time limited basis either in the hope that further trials will arise, or under a requirement of data collection (see also Hailey and Harstall, 2001). In the Australian context, the Medical Services Advisory Committee has recommended this interim funding approach in cases where: the technology is treating a serious condition; there is some reasonable and substantive evidence available to support the technology, albeit evidence that falls short of that required to support an unconditional recommendation for funding; and there are clearly definable questions relating to the evidence that is needed for future decision making combined with reasonable prospects of those questions being answered.

Controlled diffusion of technologies can also promote appropriate innovation, especially for medical technologies. In the case of pharmaceuticals, the R&D is generally complete when they come to the attention of appraisals committees. In the case of medical interventions, however, there is often difficulty in achieving an adequate level of further development following the research and widespread dissemination of the technology. Such "responsible management" of clinical innovation is the purpose of the UK's National Institute for Clinical Evidence (NICE) Interventional Procedures Programme. This programme may address some of the problems that arise under the following point.

vii. "Catch-up" evaluation of diffused technology requires case by case decision making

Tensions arise in evaluating technologies which have already been diffused in the health-care system. As Hailey and Harstall (2001) note, decision makers may *1)* be unsatisfied with the evidence for existing technologies and *2)* fail to identify innovations before they are evaluated and diffused. Increased risk of the latter happening is a necessary corollary to devolution of decision making in technologies to local administrators, where registration systems such as the NICE Interventional Procedures Programme may not exist.

The tensions arise in large part out of conflict between provider and patient expectations. The decision maker has to judge the degree to which these expectations are reasonable. To take the two extreme ends of the continuum of possibilities:

- A widely diffused technology for which there is little evidence, but where further evaluation (such as a randomised trial) is inappropriate[6]/unethical.
- A new technology that within a very short space of time is disseminated widely. However, when evaluated it is found that the evidence that exists is of poor quality.

Decision makers may wish to take a firmer stance on funding and insist on more high-quality evidence for the second case than in the first case.

viii. Develop strategies to minimise post-decision risk.

So far the discussion of uncertainty has focused on quality of evidence. However, uncertainty can also arise in the rate of take-up and utilisation of technology. Here, decision makers face uncertainty in not knowing what the likely level of technology diffusion will be once a decision has been made. Such a risk is exacerbated in health systems where funding of health services is via reimbursement or other prescribed eligibility (and hence, uncapped) programmes such as the Australian Pharmaceutical Benefits Scheme and Medicare benefits arrangements for medical services.

An approach which can be taken to address this is to establish risk-sharing agreements (between, for example, government and industry) through pricing arrangements. For example, public funding of new technologies could be coupled with price-volume agreements, where if utilisation goes over a certain threshold the price paid is adjusted. Another possibility is to agree to connect the price of a new technology to the expected level of relative effectiveness. This could be an option for a technology that can be used for multiple indications, but where the level of cost-effectiveness depends on which indication it is used for. In this case an average price can be set, weighted by the expected utilisations for each of the specified indications (Mitchell, 2002).

In addition to using price-volume agreements for specific technologies, they can be applied more broadly to cover a set of services or technologies used in specific fields of medicine (*e.g.* diagnostics). Under such an agreement overall public expenditure is limited to a certain amount for a fixed period of time – and prices are adjusted when total expenditure exceeds the agreed amount. Price-volume agreements can be designed with a degree of flexibility so that when new innovations are introduced into the package of publicly funded care, the agreed level of funding is adjusted accordingly. Such measures provide greater certainty over outlays, despite the formally uncapped nature of the financing arrangements.

6. For example, imagine there is a lack of evidence on the relative effectiveness of a surgical intervention versus "watchful waiting" for painful condition X. The surgical intervention is common, established and appears to bring instant pain relief to patients suffering from X. The question then arises: "who would agree to randomisation in a trial to answer this question?"

ix. Balance the evidence against other objectives as not all QALYs[7] are equal.

There is a lack of clear and detailed guidance on how to incorporate social preferences into decision making and how to weight evidence and economic analyses around these[8]. It is not clear, however, that taking social preferences and other objectives into account in decision making is incorrect in either a procedural or policy sense. To put it one way, efficiency is only one of many health policy objectives and economic evaluation remains the servant of policy objectives, not its master.

Consider the case when a cost-ineffective treatment targets high priority populations. It may be argued that such care is highly expensive and has potentially poor outcomes. Therefore resources might be better deployed elsewhere and deciding to provide such care may be inconsistent with other decisions. However, consistency of decisions measured by comparisons of ICERS between different interventions only holds when everything else is equal. But this is a case where this condition does not hold. This case appeals to a broader belief held in many societies that the very sick and vulnerable are worthy of special consideration, as ultimately encapsulated in the "rule of rescue". This does not reflect irrationality but rather the idea that economic evaluation exists within broader social and ethical frameworks.

This debate also touches on issues of where public responsibility of health-care ends and private responsibility starts. That is, should the public purse be used to fund all health-care services that may improve health (as measured for example through quality adjusted life years)? In Australia, for example, certain services which are carried out by way of health services – such as cosmetic surgery[9] – are not publicly funded but can be privately purchased. This is despite the possibility that individuals may hold preferences that would make these services acceptably cost-effective compared with other possible health interventions.

New and emerging technologies may make this debate even more pertinent. For example, future personalised pharmaceuticals may very well reduce the risk of side effects and increase the likelihood that the treatment will be effective. At the same time, new pharmaceuticals developments, such as "life enhancing" drugs to improve performance, or that the so-called "intelligence drugs" may have major psychosocial or even cultural consequences. These developments may add to the decision makers' uncertainty, and Professor Smith's job (from Box 7.1) may become even more difficult. These issues are taken up in Chapter 8.

7. Quality-Adjusted Life-Year (QALY).

8. In April 2004, the United Kingdom's NICE published its latest methods guide for its technology appraisal programme, which in part starts to deal with this topic. The document is entitled the "Guide to the Methods of Technology Appraisal" and will be made available on its Web site: *www.nice.org.uk*.

9. Treatment for erectile dysfunction is another local example.

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Chapter 8

POLICY DECISION MAKING IN THE FIELD OF BIOMEDICINE

Ingo Haertel (Germany), Phil Jackson and Barbara Slater (Canada)

Health-related biotechnology is developing at unprecedented speed and is delivering new techniques for preventing, treating and managing disease, but there is an extensive debate about the use of some of these new technologies. This chapter examines the specific challenges for policy makers and assessors of some of these technologies. It also sets out the additional considerations to make more informed decisions as well as current shortfalls in providing this information. Finally, this chapter suggests some instruments that may fill these gaps.

Introduction

The medical biotechnology sector has developed over the last decades at an unprecedented speed and it is clear that human health-related biotechnologies will gain enormous influence in the provision of medical care. Technologies such as genetic testing and autologous stem cell transplants have already been introduced into the health care system; others such as bio-artificial organs and gene therapy are still in the research phase and are just approaching the border of introduction into clinical use. In some cases, for example therapeutic cell nucleus transfer, the clinical use is still far in the future.

Already a significant and increasing number of applications to the major drug approval agencies are biotechnology-based products or "biologicals". For example, in 2002, the United States' Food and Drug Administration (FDA) approved some 78 new drug applications and 34 major new biological agents – as well as a further 34 biotech agents that were substantially equivalent to existing products. These biologicals include life-saving products such as recombinant vaccines for Hepatitis B, rDNA human insulin, and combination vaccines for infants.

The huge advances in understanding of the human and other genomes is delivering much of the impetus to research and develop candidate technologies. The Boston Consulting Group (BCG), for example, has estimated that by 2015 the market share of genome-based drugs will grow to 40% of the total pharmaceutical market (Tolman *et al.*, 2001), though there remains considerable uncertainty around exactly when genomics-based products will hit the market in significant numbers (the current received wisdom suggests around 2007-2010). There is also some uncertainty about how health systems will deal with this[1].

1. For example, will primary care physicians be sufficiently trained in genetics to deal with a demand for prescription of "tailor-made" medicines based on genetic polymorphisms in patients – or to prescribe the genetic tests to detect these?

Revenues from biotech-based research in the health sector are also increasing quickly, after a rather shaky start (which is testament to the commercial risks involved in much of this research). Ernst & Young (2003) estimate that in 2002 revenues for US biotechnology companies increased 15% to more than USD 33.6 billion, and R&D expenses jumped 31% to more than USD 21 billion. Tissue engineering, to take one example, is expected to grow from a market volume of USD 232 million in 2000, to more than USD 1 billion by 2007 (Frost and Sullivan, 2001).

But biotechnology goes well beyond delivery of new medicines and vaccines and tissue engineering. There is much debate in many OECD member countries about the safety, societal safeguards and ethics of using some of these new technologies. For example, for technologies such as reproductive cloning, germ line therapy and the development of "designer" babies, it is unlikely that these will ever be used in a clinical setting.

Many bodies and organisations (international and national) professionals and patients, are considering the implications of advances in human health-related biotechnology (see, for example, OECD, 1999 and OECD, 2000). Such consideration has not only been give to safety, society and ethics, but also to creating the conditions for innovation to deliver improved health outcomes more effectively. For example, the Ministers of Science from OECD countries[2] adopted a report on "Biotechnology for Sustainable Growth and Development" (OECD, 2004) in January 2004 that identifies genetic science and biotechnology as key drivers for economic development and better health outcomes in their countries.

Parallel to the development of medical biotechnology, decision-making processes in the health care sector have been systematically improved. The structured analysis of the scientific soundness and the individual and systemic economic impact of new technologies are today a standard requirement in the preparation of policy decisions. Health Technology Assessment (HTA)[3] strives to provide such information to decision makers and the use of this information is increasing.

However, as outlined in previous chapters, the HTA process itself faces some significant challenges. HTA results appear to be more valuable for some techniques, namely pharmaceuticals or medical equipment that replaces existing technology, but less valuable for technologies that are at earlier stages of development or that can be expected to have a more complex impact on health care and society.

From the perspective of decision making about the use of technology, many of these new biotechnologies present additional complexities (*e.g.* allocation decisions for orphan drugs and tailor-made medicines; how to take early account of stakeholder perspectives) even when considered within a defined overall policy framework[4]. Developing a defined policy framework has often lagged advances in human health-related biotechnology, so

2. As well as those from China, Israel, Russia and South Africa.

3. HTA is referred to in this as assessments done through both formal HTA agencies and those conducted by drawing on available expertise, including that of private sector entities. There is recognition that countries evaluate technologies through a variety of mechanisms and in this paper the use of "HTA is meant to be inclusive of those differing mechanisms and institutions.

4. A defined policy framework could be, for example, one in which there is an explicit policy to encourage and reward the introduction of innovative medicines and help pay for this by encouraging maximum competition in off-patent and generic markets. Reimbursement and market access policies and specific decisions on technologies might fall within this overall policy context.

that decisions about such technologies have been made without an established policy context within which to operate. As noted in Chapter 7, however, even when there is no extant policy framework some decisions are taken, even if only implicitly.

This means that there are significant challenges for decision makers to make informed and acceptable choices so that there is optimal uptake of the respective technologies. This chapter argues that in policy decision making with regards to value laden innovative biotechnologies, the current approaches in preparing health policy decisions, which are focused on cost-effectiveness analysis (CEA), have to be supplemented by other means that go beyond the economic evaluation and may include ethical, social, organisational, legal and macroeconomic aspects. In order to develop these aspects, an internationally co-ordinated effort should be made.

This chapter also argues that the adequacy of current HTA analytical tools will be challenged by some of the characteristics of innovative biotechnologies, and that current approaches in informing decision making could be enhanced by other means that include relevant ethical, social, organisational and legal aspects, even (or perhaps especially) in the absence of established policy frameworks.

This chapter examines a number of the characteristics of certain health-related biotechnologies that create very specific challenges for policy makers and assessors of technology. Also examined are the types of decisions that policy makers are coming to terms with in the field of biotechnology, and the information requirements to make more informed decisions. Finally, current shortfalls in providing this information are identified and instruments that may fill these gaps suggested. The analysis concludes that further international co-operation might bring added value to the development of supplementary toolkits for more informed decision making in certain human health-related biotechnologies to improve the efficient and effective use of these promising and important innovations.

What is special about medical biotechnologies?

While numerous features of medical biotechnologies are comparable to "conventional" medical technologies, a number of new challenges arise and many are more acute than has been the case for other medical technologies. From a decision making perspective, this sets some kinds of human health-related biotechnologies apart. The main challenges include:

Speed of development

The speed of development of many human health-related biotechnologies has increased substantially with automation, better understanding of informatics and a move towards increased technology convergence (particularly with the information technologies). There are a number of examples of the introduction of such new biotechnologies into health systems through applied clinical research rather than according to the familiar "top to bottom" model, which is particularly common in more centralised health systems and where there are fairly well established controls on supply and demand.

Application of diagnostic genetic tests is a case in point, where clinicians were often using tests before commercialised diagnostic products were actually marketed. Consequently, there was little or no formal consideration of clinical and cost-effectiveness as provided through an HTA. Patient demand for some tests rose rapidly, even though there remained uncertainty about their appropriateness, quality assurance, and predictive

ability[5]. As discussed in the previous chapter, decisions needed to be taken – implicit or explicit – despite the lack of good evidence.

In other cases, the speed of advance of biotechnology has meant that technology has led policy development rather than *vice versa*. Use of stem cells and therapeutic cloning are good examples here. Potentially, the diffusion of useful human health-related biotechnologies is impeded by the relatively slow response rate of public policy debate. No decision means a "no" decision in such cases.

Investment risks and returns

Very large investments have been made by public and private sector alike in pushing forward understanding of disease, in particular under the auspices of the human and other genome projects. A number of medical applications are arising from this investment and many more are expected. There is considerable pressure on innovators to commercialise such applications, both in the public sector, where some return on research investment may be sought, and in the private sector, particularly given the prevailing business model of small to medium sized dedicated biotechnology firms looking to secure income streams based on sales rather than only investment capital.

Against such a background, timing of market entry and market uptake can become of critical importance to the financial viability of many businesses. If decision makers are unprepared to make a "yes" or "maybe" decision due to a lack of good analytical evidence, or a clear policy framework, the consequences may be significant for the sustainability of innovators and supply of innovative products. Early dialogue between innovators and decisions makers – as advocated in Chapter 4, may help avoid getting to a "no" decision based on lack of understanding or evidence and so help minimise investment risk.

High cost and effectiveness

Allocation issues, which are common in most sectors of the health-care system, can become particularly visible with biomedical technologies where highly effective treatments for a very few are available at very high costs. This can be perceived as stretching notions of solidarity in a public health insurance system, or create greater risk selection problems under private health insurance.

An example of this is Fabry's Disease, an inherited metabolic disorder that affects one in 40 000 people. Fabryzyme is a highly effective treatment for this disease that costs approximately USD 200 000 per year per patient. The cost of such treatments could absorb a considerable amount of the health-care spending of any given system, though the benefits are very considerable.

This "allocation dilemma" needs to be viewed at several (often competing) levels, including the level of the clinician as opposed to the population level and societal level. While this is not unique to biotechnologies[6], such products, especially for orphan

5. A good example of this was the introduction of the BRCA 1 and 2 tests for predisposition to hereditary breast and ovarian cancers. Despite significant debate about the appropriateness of the test and controversy over the benefits of it, the commercial test was introduced in 1998 (in Canada) and demanded by patients and used by clinicians

6. The allocation dilemma is perhaps more pronounced here simply because biotechnology has successfully delivered treatments for such rare and serious diseases.

diseases, may be launched earlier than "mainstream" products into small fragmented markets where the clinician plays a particularly strong role in disease management.

The issue of highly efficient yet costly treatments for such rare diseases has already led to consequences in the pharmaceutical marketing of innovative drugs. At the present time, there are considerations in some countries to make patients pay for participating in phase two and three studies where there is justified hope for therapeutic efficiency.

Standard methodologies applied in health technology assessment may struggle to deal with such cases and there is no agreed proven system in place that can assist the decision maker to make appropriate allocation choices for rare diseases (Danzon and Towse, 2002).

It is possible that these issues around allocation will be brought into even greater focus with the expected onset of "tailor-made" pharmacogenetics-based medicines. Such treatments hold the promise of greater effectiveness, but higher costs (partly since the target population for such treatments will by definition be smaller than for equivalent "conventional" medicines).

Decision makers thus will face two main challenges with such developments. First, how to encourage those carrying out HTAs to take account of the differential effectiveness of technologies within different polymorphic groups and, second, whether and how HTA methodology might evolve to value some health benefits (for example, children with rare metabolic diseases) more than others.

The reach of genetics-based technologies

A number of the new health-related biotechnologies involve eliciting genetic information about individuals (*e.g.* genetic testing), addressing genetic defects (*e.g.* gene therapy), or otherwise using genetically altered material for health benefits (*e.g.* certain applications of xenotransplantation).

Whereas such genetics-based biotechnologies promise much by way of potential health benefits, they also give rise to some peculiar challenges. For example, though there are established privacy and security practices in place for handling medical data, genetic data is subtly different since knowledge about an individual's genetics might not just benefit that individual but also benefit the progeny, siblings and other family. Furthermore, genetic information has the potential to be predictive of disease – again such information can be of positive and negative value to more than just the individual patient. Finally, there is a potential temporal dimension to genetics and some genetics-based technologies that sets these apart from many other health-related technologies.

The more general issues around privacy, security and access are important conditioners of the policy framework within which decisions are taken. The predictive ability of individual tests (over which there may be some considerable uncertainty), and the effectiveness of genetics-based interventions, are of direct concern to decision makers. Furthermore, the increased genetic knowledge can alter our perception of disease and thereby create new challenges for health care systems.

Ethical and public perception considerations

Some biotechnological techniques (for example, therapeutic nuclear transfer, human embryonic stem cell applications and genetic screening) are ethically controversial, raising issues around the concept of life. The use of human embryonic stem-cells for research purposes either derived from embryos created but no longer used for in-vitro

fertilisation (IVF) projects or created intentionally, raises questions for some about human dignity and the right to life, as well as freedom of research. Other examples of where biomedical technologies potentially challenge ethical values in many countries are:

- Privacy and confidentiality of genetic information (*e.g.* use of genetic information by a third party).
- Start and end of life (*e.g*. use of foetal material and termination of pregnancy).
- Value of information and the use of information to make choices that may be ethically problematic (*e.g.* termination of pregnancy, partner selection or selecting donors such as siblings and testing of young people with untreatable disease).

Of course, many other health-related technologies and practices raise ethical issues (for example, techniques of *in vitro* fertilisation, pregnancy termination). The importance of the ethical perspective can be illustrated with the example of therapeutic nuclear cell transfer (therapeutic cloning). The position on this issue taken by different OECD member countries differs significantly, although the factual basis is reasonably clear and the perceived future benefits are similar amongst countries. Nevertheless, the differing positions and conclusions on this issue are highly visible and international consensus seems far away.

Part of the rationale for taking different positions on some human health-related biotechnologies is that countries adopt different principled ethical positions, but part, too, is down to a political decision reacting to perceived public opinion. In many if not most cases, it is not possible to separate out the extent to which these two issues influence decisions on the prevailing policy climate.

What can be said is that biotechnology has caught the attention of the public in ways that most other medical technologies have not. A number of studies (Tambor *et al.*, 2003; Priest, 1999) have shown that people are simultaneously enthusiastic about the benefits from biomedical technologies yet concerned about the perceived potential harms from them, such as concerns about misuse (of, for example, reproductive human cloning). For instance, the public is more concerned about information that is provided through genetic testing than other sources of medical information (Tabor *et al.*, 2002; Jallinoja *et al.*, 1998; Laskey *et al.*, 2003). It is possible that such concern could become even more pronounced as future genetic tests are applied to the major disease groups and become more common.

However, although public interest is intense, the general public often only has a limited understanding of the scientific background as well as of the economic implications and issues. This divergence between interest and understanding may lead to exaggerated hopes and exaggerated anxieties.

Decision makers are thus faced with a particularly complex political environmental in which to take decisions about human health-related biotechnologies. The multiple causes for public attention to these technologies need to be taken seriously if decisions on their use are to be widely accepted by all the stakeholders concerned – including the public. Getting this context right will be essential if the situation is to be avoided whereby no decision translates into a "no" decision and promising technologies and the health benefits they could provide fail to reach patients.

The key challenges for decision makers

The perceived economic opportunities and the resulting demand for reliable policy decisions to provide a predictable developmental environment for these technologies, coupled with intense public interest, have created intense pressure on decision makers to take positions both on specific technologies and on the wider issues raised above at an increasingly early stage.

As with other health technologies, the current methods of HTA are helpful in preparing policy decisions where criteria like scientific and clinical validity, clinical utility and cost-effectiveness are relatively clear cut. In general, the less circumscribed a decision is by factors outside the cost-effective analysis, the more helpful HTA can become.

However, the characteristics of human health-related biotechnologies set out above mean that some decisions in the field of biomedicine are particularly difficult to make, because the factors that need to be considered may be broader than scientific and clinical validity, clinical utility and cost-effectiveness and may lean particularly heavily towards societal factors (such as patient acceptance, or views on spending allocations). Some of these issues can be addressed inside the HTA "box"[7] whereas others are clearly part of the consideration of the wider policy framework.

Box 8.1. Gene therapy:
A case study in the impact of uncertainty on innovation in health biotechnology

Decisions taken in the health-care system have implications on public and private R&D investment, and choices about R&D strategies impact on the delivery of health-care system goals. The case of gene therapy illustrates this. The first successful clinical trials created a fruitful and highly expectant atmosphere for research in this field, before there were clear research results. However, subsequent clinical trials of gene therapy showed that there were unexpected risks associated with the technology. Early expectations from patients were in many cases disappointed and as a result there was a crisis of trust between investigators and patients. Many decision makers reacted to this by withdrawing support from gene therapy work. The impact of this has been to stifle innovation at a critical point in what had been regarded as an extremely promising nascent technology sector. Earlier attempts to address patient expectation and to ensure their involvement in the process towards decision making may have helped lead to a more favourable outcome.

Decision makers of course have a responsibility to make well-informed choices. It is suggested here that given the challenges posed by human health-related biotechnologies there is a comparatively greater potential for decision makers to make ill-informed decisions than in other areas of health technology. The research and development behind many fields in biomedicine is still in its relative infancy, and may therefore be particularly vulnerable to such ill-informed decisions. Today's decision (even when the decision is to do nothing) will impact tomorrow's scientific direction and, as a result, may stifle future scientific discovery (see Box 8.1).

As for other areas of health technology, decision making can be split broadly into three levels – macro, meso and micro[8].

7. See Figure 1.2 in Chapter 1.

8. These are explained in some detail in Chapter 1.

All levels of decision making are important, but the real challenges for decision making about health-related biotechnology tend to fall to the macro level; for example, broad questions about issues such as screening programmes or decisions about the inclusion of predictive tests into the health-care system are often taken at a national level. Similarly, HTA methodology questions (on dealing with challenges from pharmaco-genetics or orphan medicines, for example) might also be dealt with at the macro level.

Development of helpful tools and approaches

Many of the challenges that health-related technologies pose for good preparatory work in the field of biomedicine are shared with other types of technologies. Indeed, most of these challenges are well within the tradition of HTA and do not constitute new challenges for the decision-making process. But in many cases the characteristics of biomedical technologies (*e.g.* value laden technologies that are under public scrutiny) result in extended requirements for the decision-making process.

There are many potential techniques for gathering the type of information needed for good preparatory assessment in the field of health-related biotechnology decision making. A number of these techniques are listed here, although some require considerable methodological development, and an analysis of which techniques are most effective is also needed. This section looks at additional preparations that can facilitate making more informed choices on health-related biotechnologies.

Ethical, social and legal issues

The potential ethical implications of the technology and its use, both in the health system and in wider society, need to be understood. Any such implications should be considered by existing ethical frameworks, such as those developed by ethics boards for medical research, statutes of rights and obligations of citizens as well as international treaties and declarations.

As with many other health-related technologies, biotechnologies may sometimes have social consequences. For example, some biotechnologies may be able to deliver better health outcomes for a small number of patients suffering from debilitating diseases but at a very high price. In such a situation, as for other health-related biotechnologies, a more clearly defined concept of equity, as well as more information on what trade offs between efficiency and equity are socially acceptable, would be useful to decision makers.

Any ethical and social consequences of some health-related biotechnologies may be reflected in public opinion. As such, good preparatory assessment might not only be restricted to the "hard" facts (*e.g.* scientifically proven evidence) but also develop an increasing role for the "soft" facts in the decision-making process. Any such "soft" information might, for example, be gathered through citizen conferences, surveys, expert opinions and panels.

Any such preparatory work might usefully consider public attitudes and develop a clear assessment of which aspects of the technology in question are controversial, uncertain or inconsequential. Such preparatory work might *inter alia* undertake some scenario building, where the consequences of various policy options are analysed.

Such technologies and the consequences of their use may raise questions of legality and liability (for example, regarding privacy of information). An assessment of the impact of current legislation on potential use of such technologies could be helpful to decision makers.

Timeliness

The potential ethical implications of a technology highlight the need for timely preparatory work prior to decision making. Without good early preparatory work, there are greater risks for innovators and society. The risk to innovators is that decisions could be delayed or a negative decision is taken because of incomplete information. The risk to society is that a "wrong" decision could be made. For example, decisions can be unnecessarily delayed, a technology restricted because of a mistaken perception of an ethical issue, or a technology disseminated where there are ethical implications.

The need for timely preparatory analysis for technologies that have ethical implications suggests that there may be a case for supplementing existing models of assessment.

Dissemination and communication

The need for better dissemination techniques for the preparatory analysis is emphasised by health-related biotechnologies. For these technologies, preparatory assessments have to inform not only health-care decision makers, but also a wider set of stakeholders. This is because health-related biotechnologies often face higher public scrutiny. In order to develop greater involvement and education about the likely impact of the new biotechnology, dissemination of evidence should be encouraged to a wide range of stakeholders including clinical provider associations, media and patient organisations.

Future dissemination strategies could support integration with broader types of knowledge that are needed to round out the information provided through traditional HTA.

From static to dynamic assessments

Currently, HTA is often undertaken, particularly by public authorities, at the time when the technology is ready for clinical application and the market. HTA usually focuses on the scientific evidence from randomised controlled trials, or other scientific studies, as a way of assessing evidence which corresponds to information on costs and efficacy. Hence, technologies are assessed towards the end of the R&D cycle, the evidence is then appraised and a decision taken.

Such an approach to assessment takes a static perspective of the innovation cycle and can overlook the dynamics of further innovation of a technology. It can also mean that no consideration is given to any societal impact of a technology until it is available for launch. Subsequent uncertainty amongst decision makers may to lead a delay in clinical use. The static perspective thus might be regarded as risky.

Figure 8.1 presents this static assessment as Model 1. A more dynamic method of assessment, appraisal and decision making is set out in Model 2. Here "assessment"-whether carried out by an innovator or third party – and some form of "appraisal" is carried out more than once across the innovation cycle, and the results fed back into R&D. This model is as applicable for early identification of any societal consequences of

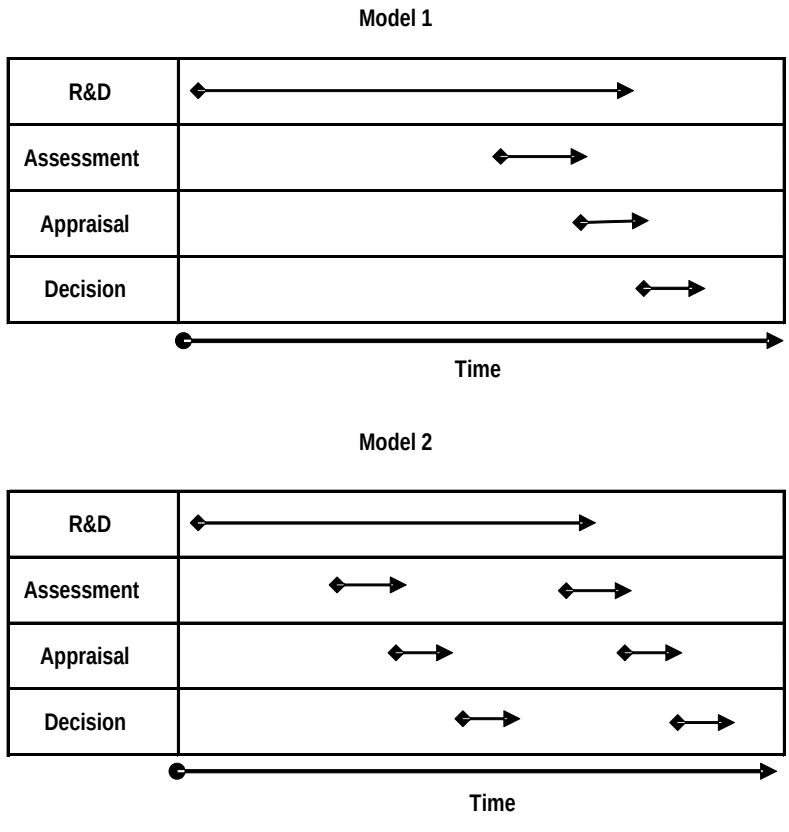
a technology (thus avoiding delay in launch) as it is for adapting to post-market innovation (see chapter 7).

There is some limited evidence of development in this area. For example, in the field of public reimbursement processes, more emphasis is being placed on the issue of post-marketing surveillance (see Chapter 4). A key objective of this is to generate real-life evidence by which health planners, health funders and clinicians can accurately evaluate the degree to which the claims made about a certain technology can be justified by long-term clinical evidence. Such mechanisms could also potentially be used as a signal process to the R&D field. To date however, experience with such surveillance in the public policy context is limited.

Systems that foresee the emergence of a technology may aide in a better understanding of the technology (and its potential implications) at a very early stage. Methods of doing this include horizon scanning activities and emerging technology bulletins. Anticipatory advisory boards have already been set up in a number of countries to maintain a watching brief on technologies and their development.

This is an important issue for many health-related technologies, including biotechnology. Small to medium sized firms, as commonly found in the biotechnology sector, are susceptible to delays in market entry.

Figure 8.1. Static and dynamic models of assessment



Valuation of benefits

HTAs, and more specifically cost-effectiveness analysis (CEA), have also been criticised for the methods used to value a technology's benefits. This is of crucial importance in a number of areas such as genetic testing (Col 2003, Giacomini *et al*. 2003, Hall *et al.*1998). Like any diagnostic test, the product from genetic testing is information. Subsequent treatment decisions, based on the information given by the test, may deliver additional health outcomes (*e.g.* additional life-years). However, information from genetic tests may also be of value (both negative and positive) to the patient and their family (*e.g.* reassurance) and may also impact on social interactions and relationships (*e.g.* choice in reproductive partner). Until evaluation techniques can encompass these wider notions of benefits, assessments will be either incomplete or misleading. (Hall *et al.*, 1998).

Societal concerns about fairness are also a key consideration for the valuation of benefits from health-related biotechnologies. The cost per treatment can be very high for some biotechnology-based treatments due to high R&D costs and the small patient groups. At the same time, these treatments can benefit patients who: *1)* may be severely ill; or *2)* represent a high-priority population group (*e.g.* children). For any of these reasons, there may be a strong equity consideration in the valuation of benefits.

There are ways in which societal preferences can be incorporated into the valuation of benefits (Morone *et al*, 2003; Nord *et al.*, 1999). Such approaches aim to bring societal notions of equity into the valuation of health benefits, through, for example, surveys on values and trade-offs. The measures derived from such surveys can then be incorporated into a more just valuation of health programmes that take into account fairness as well as efficiency (Nord *et al.*, 1999).

Considerations for health-related biotechnology decision making

A question arises as to "how" any additional considerations for preparing decisions for specific biotechnologies can be undertaken, but needs to be separated from the question of "who" is going to provide such information to decision makers. An important issue is whether these tasks can be incorporated within HTA or whether other means of assessment should be used.

One argument for diversification of the preparatory "assessment" process before decisions are taken is that there is a high degree of competence in the technology assessment relating to economic and epidemiological and effectiveness issues but experience outside of these realms is often lacking. It may be of greater benefit to give separate tasks to separate specialists, asking HTA practitioners to do what they do best, and search for expertise on ethical, legal and social issues outside of HTA.

However, there are examples where HTA agencies have conducted extensive appraisals on various societal aspects using participative methods (*e.g.* focus group interviews, Delphi processes and public forums)[9]. Qualitative methods and the broader application of HTA are increasing but not yet widespread. Methods are in development to research the ethical, social and legal aspects of new technologies. Whether those means should be applied by HTA agencies or by others depends on the structure of the respective policy making processes.

9. See, for example, the British Columbia Office of Health Technology Assessment (2000).

A further model is for HTA institutions to seek and integrate general technology assessment expertise where specific participative methods are needed. A network of general technology assessment institutions exists in Europe (European Parliamentary Technology Assessment Network, EPTA).

However, questions remain concerning whether HTA specialists are willing to undertake this expanded set of tasks, or whether they believe HTA (as it may be practised in their country) should stay focused on the "hard" scientific evidence. There may be an argument for HTA to retain its competency in validity and economic questions, but leave "soft" qualitative analysis to outside bodies. From the perspective of the decision maker, transparency, impartiality and objectivity are basic requirements embedded in today's understanding of democratic societies. To fulfil these requirements, it seems advisable not to concentrate all preparatory tasks in one institution, even if such an agency refers in their assessment to multiple outside sources. This is because it is still one agency's interpretation of the facts, the uncertainties and the social impacts.

That said, it is evident that HTA is in itself a very diverse field and there are as many different ways of involving HTA in decision-making processes as there are countries doing HTA. So it is probably appropriate to leave the decision on how to proceed to the individual country or health decision maker, as long as all aspects of assessment are covered, it is of high quality, and involves cross-disciplinary co-operation.

Conclusions

The collective characteristics of health-related biotechnologies have been shown to complicate the existing methods of decisions, including the speed of innovation process, the investment market, close relationship to some basic values and public interest. These characteristics point to some additional considerations in conducting preparatory work. The first area is in methodological refinements of HTA; however, this task should be undertaken by national and international HTA bodies.

The second area is in the development of toolkits and good decision-making practices. Further international research should be initiated that concentrates on filling the gaps that are apparent in the process of policy decision making in the field of health-related biotechnology. The development of good practices and toolkits could be seen as analogous to the development of HTA standards.

Such work could be focused on helping decision makers make good decisions, regardless of the form that their technology assessment process currently takes. The development and analysis of best practices and toolkits would help to improve the alignment of individual health system objectives with specific decisions, as well as improve the decision-making process itself. To achieve this, a close relationship is needed among all stakeholders who can contribute to the development and use of biomedical technologies.

In scanning the international circle of decision-making processes in biomedicine and beyond, it becomes apparent that many different approaches exist to incorporate the spectrum of the aforementioned requirements for policy decision-making processes. However, whilst the outcomes of policy-making processes have undergone international (mostly legal) comparisons, the tools and practices of the policy-making processes themselves have not. More structured multidisciplinary international exchange is seen as a valuable and efficient approach to fill this gap. Individual countries simply cannot be expected to run trial and error research in this sensitive policy field.

Some of the special characteristics found in many health-related biotechnologies can also be found in other innovative technologies. This being the case, it is evident that some of the results of the proposed work could be applied, for example, to value laden non-biomedical techniques. The results of a study in the field of biomedicine might also be considered of value beyond technology decision making. For example, the growing financing and allocation problems in all developed health-care systems share many of the same, described characteristics. They are under public scrutiny, they touch basic values, and they are, for a variety of reasons, only to a limited extent accessible to traditional HTA approaches focused on specific technologies. It can therefore be expected that the answers found in further studies regarding good preparatory analysis for policy decision-making practices with regard to biomedical technologies (*e.g.* decision-making guidelines, best practices) might also lead to a payoff in a larger spectrum.

The set of activities outlined above could be appropriately dealt with within the framework of the OECD. It has already carried out work on evidence-based policy making and decision making in health-care systems, and its consensus framework would enhance the development of toolkits. Furthermore, it is apparent that in light of the ever-expanding internationalisation of technology driven industries, medical research and clinical practice, best practices and toolkits will provide greater value if developed at an international level.

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Chapter 9

CONCLUSIONS

Health-care innovation has transformed the practice of medicine over the course of the last century. Technological innovations can bring substantial benefits in the prevention, diagnosis and treatment of disease, as well as access to care. At the same time, there is widespread variation in the use of health-care technologies across OECD countries, indicating that the most effective and efficient technologies may not always be the ones most often used. Encouraging the uptake of efficient and effective health-care technologies is a significant policy challenge in many OECD countries.

This OECD study focused on the way decisions about the uptake and diffusions of technologies are made in health-care systems. It has aimed to provide evidence on how OECD health-care systems can enhance decision making on the use of technology. It examined the production of evidence, primarily in the form of health technology assessment (HTA), and the use of evidence in decision making. In doing so, it has focused on the transition from the production and synthesis of evidence, to the appraisal of evidence, to decision making and decision implementation.

The challenge for many policy makers is to develop policy instruments that lead not only to the optimum levels of diffusion or use, but also encourage development of technologies that match priorities. However, efficiently integrating technology into the health-care system is complex because many thousands of decision makers collectively influence technology diffusion and uptake. The task is made more difficult by the fact that some health-care decisions translate directly into decisions about who gets what care, when and on what terms. Such decisions carry complex analytical issues straight onto the radar screens of public opinion and a myriad of organised groups.

The focus of this study has been on decisions typically made at national, provincial or hospital levels, often referred to as macro and meso levels[1]. The conclusions reported here draw on five case study technologies that illuminate HTA production, decision making and implementation in 12 participating OECD survey countries. They also draw on evidence from the literature and discussions at the workshop on health technologies, held in The Hague on 27 and 28 October 2003. This chapter presents details of the policy implications of the study and sets out some important challenges for decision makers.

1. The results of this project reflect the perspectives of decision makers within the context of macro and meso decision levels. Care should be taken not to generalise the results of the survey beyond these contexts.

Before turning to the detailed conclusions of the study, the key findings are:

- HTA is of significant value in aiding evidence-based decision making. It needs to come to terms with some significant challenges in the delivery of timely and relevant information that reflects the dynamics of the technology and the health-care system.
- The ways in which health-care decisions are made require greater clarity, transparency and to be more conducive to the incorporation of evidence.
- Greater stakeholder involvement can facilitate improved implementation of decisions and policy, and manage uncertainty whilst enabling access to safe, effective and efficient technologies.

Policy implications

The production of evidence and health technology assessment

Better management and integration of health technologies require accurate and timely information about their effectiveness and costs. In the absence of such information, the uptake and diffusion of technologies are more likely to be influenced by a range of social, financial, professional and institutional factors and may not deliver the best possible health care. Therefore, a vital condition for making informed choices is to have access to evidence and to have strategies in place for those situations where detailed evidence is lacking.

OECD countries are recognising the need for greater evidence-based decision making in health. The production and dissemination of HTA[2] is a key component of this recognition and many countries have seen increased levels of investment in HTA-related activities, including methodological developments and greater application of information technology to disseminate HTA findings.

In many OECD countries HTA is already an important component of both public and private sector decision making. It is expected to play even larger role in the future. The dynamics of medical innovation, as well as recent scientific advances, are likely to increase the pace of technological development. Being required to keep-up with the opportunities and challenges created by new technologies, decision makers will need access to more high-quality synthesised evidence. Furthermore, the common trend to devolve health-care decision making will mean that more and more decision makers require access to such evidence. The value of HTA is therefore likely to increase but there remain some significant challenges that need to be overcome if HTA is to fulfil its role in driving greater use of evidence in decision making.

Clinical evaluative research and HTA account for very small proportions of total health-care spending. Developing a culture of evidence-based medicine and policy requires secure and long-term investment to ensure the development of analytical capacity and expertise. New partnerships (amongst, for example, government, industry,

2. HTA considers the broader impacts of health technologies and typically involves *1)* identifying the policy question, *2)* systematic retrieval and analysis of scientific evidence, and *3)* appraisal of evidence, including judgements about the evidence. The evidence and appraisal then inform decision making.

public R&D organisations, and insurance institutions) that invest in such research may help share the cost burden.

Co-operation and communication amongst HTA producers, users and other stakeholders is essential to ensure more comprehensive assessments of a wider set of technologies, reduce potential duplication and ensure that assessments are in line with decision makers' priorities. Such models of co-operation also need to reflect the local HTA production capabilities and institutions.

At the same time, the production of HTA varies considerably among countries and this is to be expected. HTA has evolved within the framework of specific OECD member country health-care systems. Just as health-care systems differ, so does HTA. In fact, the adaptation of HTA to the individual characteristics of a health-care system can deliver more relevant information to the decision-making structure of that system, and enable the interaction between scientific evidence and local values.

Generating greater awareness and use of evidence

Much can be done to optimise the use of evidence and HTA in decision making. The responsibility of achieving this must be shared amongst stakeholders including policy makers, decision makers, innovators and the producers of evidence. For example, to create a better awareness of HTA results, significant proportions of HTA activity need to be devoted to the dissemination of results. Whilst more research is needed to develop best practice in dissemination techniques, there are some indications that a wide range of dissemination strategies may be more effective than a single one.

More can be done to facilitate the use of HTA in decision making. Some ways that OECD countries could respond to this challenge include:

- To generate greater acceptance and appropriate use of HTA (and generally build a culture of evidence-based policy and practice) greater efforts are needed in the area of building decision makers' skills in interpreting and analysing evidence, and establishing information infrastructure to make evidence more readily available.
- HTA use can be enhanced through the development of clear decision-making processes, with perhaps a better defined function for HTA. Clearer processes will enable HTA producers to disseminate results more effectively.
- HTA has to meet decision makers' needs. This means ensuring the timely availability of information, in line with decision priorities, and recognising the various dynamics of different technology markets and technology developments. Regular updates of HTA, in line with developments in research and technology innovations, provide a means of delivering up-to-date information, although current HTA resources may make this task difficult in some OECD countries. Earlier communication between the producers and users of HTA will also be important in delivering more valued and relevant information.

Methods of decision making

It is increasingly recognised that the way in which a decision is made is an important factor in generating greater acceptance of that decision by stakeholders. Decision-making processes that are transparent, based on evidence and incorporate a review mechanism can enhance broader stakeholder support for decisions. In turn, broader stakeholder support is vital to the successful implementation of decisions.

Decisions that influence the uptake and diffusion of technology are made at all levels of the health-care system, ranging from central decisions about coverage, through to individual patient/doctor choices about which medicine to prescribe. Within the context of individual OECD member country health-care systems, each level of decision making has its own strengths and weaknesses. Local level decision makers, for instance, might be better placed to consider the suitability of the technology to local patient needs, but higher level decision makers might be better placed to consider efficient co-ordination and compatibility of health technologies used throughout the health system. A better understanding of strengths and weaknesses of decision-making levels within each health-care system and how they reflect the challenges posed by specific technologies may ensure more effective decisions.

Implementing decisions: barriers, facilitators and innovative policies

A barrier to the efficient adoption of technologies is silo funding where decision makers are responsible for their own budget and have few opportunities (or incentives) to take a wider health system perspective. Under some arrangements this will mean that decision makers may face disincentives to maximise health gains or efficiency because they are unable to transfer resources across budgets. There is a need for a greater understanding of the impact of incentives on efficient purchasing. There is also a need to identify methods which align incentives with evidence and health priorities.

The use of high-quality and trusted evidence is an important factor in the successful implementation of decisions, as is greater flexibility of resources and assistance in financing. However, implementation tools also need to be better aligned with the best available evidence. This includes developing or setting policy levers that either create incentives, or neutralise disincentives, for decision makers (in all parts of the health-care system) to incorporate evidence into their choices.

In recent years, many countries have moved away from blunt supply side controls for technology use and towards policies that place greater emphasis on value for money, measurement and accountability. As such, more OECD countries are looking to policy tools that aim to achieve multiple health system goals simultaneously. Some of these innovative policy instruments have started to recognise the value of new technologies, facilitate access, make greater use of evidence in decision making and deal with uncertainty. Whilst there is a need to evaluate these new mechanisms fully, they offer some prospect for improved integration of new health technologies into health-care delivery.

A number of OECD countries, for example, have started to introduce risk-sharing mechanisms that enable access to markets and reimbursement funding, but also create greater financial certainty for payers. Cost and volume agreements between governments (payers), professional groups and industry are one example of such mechanisms; these have already been used in various forms in a number of countries.

Similarly, decision makers in a number of OECD countries can authorise access (or funding) to technology on a conditional basis, enabling limited access to technology on a trial basis. The aim of conditional approval is to gather further information about the technology to overcome key uncertainties. At the same time, it minimises many of the risks associated with widespread diffusion of a technology of uncertain value. Such policies also recognise the dynamics of medical innovation and can have built in feed back processes for innovators, based on ongoing assessment of the technology in question and so prevent costly delays in bring technologies to the market.

However, successful use of conditional approval depends on further data gathering. This not only requires resources but also a commitment from stakeholders to agree to a procedure to collect a minimum data set that will provide additional data around the key decision-making criteria. Moreover, decision makers must be able to re-evaluate their decision, not just in theory but also in practice. This would appear to require all stakeholders to agree to a process and commit to the final outcome of the decision.

Challenges for HTA and decision making

In undertaking this analysis, many challenges in the area of decision making and HTA have been identified. This final section sets out some of these ongoing challenges and points to some possible directions for further policy action and research.

Framework for analysing the impact of HTA

With greater investment in the production and dissemination of evidence to a wide variety of decision makers, it is important to ensure that these processes are effective and efficient. To this end, there is a need to develop a more cohesive framework for analysing the extent to which HTA has contributed to making rational choices, including indicators to measure how HTA has:

- Influenced decisions in health care.
- Influenced the use and diffusion of technology in line with evidence.
- Influenced health outcomes, access and efficiency.

A framework for analysis would create greater opportunities to develop best practices in the production of HTA and in encouraging the use of HTA in decision making. If such a framework were to be internationally agreed and applied there may be further scope for understanding the impact of health system characteristics (such as, for example, payment mechanisms) and develop better practices in the application and use of HTA in both health care and political decision-making processes from a wider set of experiences.

Challenges for economic evaluation in decision making

Economic evaluation, often a component of HTA, faces its own set of challenges. One of these challenges is the potential lack of transferability of evaluation studies from setting to setting; this is in fact one of the primary reasons that economic evaluations are often not used in local decision making. The variables used in an economic evaluation may differ from those of the decision maker's local circumstances. Empirical research has shown that the way most economic evaluations are reported makes it difficult for local decision makers to assess whether the study does (or does not) bear relevance to the local setting.

Current approaches to health services research also often precludes policy makers from determining which subgroups within a study might benefit most from an intervention. This is usually due to study design, with its emphasis on measuring the effect of the intervention on an "average" population. Although less-advantaged populations can often benefit the most from health-care spending, current standards in technological research fail to give decision makers the arsenal of data needed to enact policies that deliver more equitable and efficient health outcomes. This issue will become a bigger challenge as pharmacogenomics-based products come on line.

Patient preferences are likely to become more important in determining the value of new technologies. For example, patients may prefer one technique over another, even when the expected health outcome is the same. Measures of patient preferences could be used alongside measures of health outcome and quality of life, but methodologies, including measures of patient preferences need to be better understood and defined by users and producers of HTA. A subsequent challenge will be to incorporate such information in the decision effectively.

These issues call for further methodological developments in the conduct and reporting of economic evaluations. Developments in this field should enable decision makers to assess the relevance of economic studies to local circumstances, including the applicability of the study to local population needs and circumstances. One possible way of moving forward is to develop agreed standards for the reporting (and perhaps conducting) of economic evaluations. However, agreeing on standards will not be an easy task. There are likely to be conflicting opinions about what those standards should be. Furthermore, any standards need to be able to respond to the dynamics of methodological developments.

Better transformation from assessment to appraisal

There is a need to distinguish between assessment (the objective analysis of the impact of technology) and appraisal (the interpretation of objective analysis). Both assessment and appraisal activities are important in the formation of decisions that are acceptable to society and in line with evidence. However, there remains an inevitable tension between value judgements and objective analysis. Agencies such as the UK's National Institute for Clinical Excellence (NICE) are coming to terms with this problem and have put in place enviable standards in their appraisal processes that draw on scientific and social values.

HTA can deliver information ranging from highly technical matters such as efficacy and cost-effectiveness, through to analyses of the equity, social, ethical and organisational impact of technologies. However, these latter issues are not always part of HTA, and indeed are sometimes deliberately avoided by assessment agencies that consider them outside their role and expertise. Moreover, many policy makers might be unwilling to accept such "soft" analysis from the same source that provide them with technical analysis, as that would lay them open to excessive influence from that one source. Indeed, some might argue that even the provision of the more technical kinds of information are influenced by a limited range of perspectives (*e.g.* those from the field of public health rather than from innovators), and that the checks and balances of alternative analyses are therefore a necessary part of the system. The question, therefore, of how and where - along the line from assessment to appraisal to decision making - the full breadth of perspectives on new technology should come into play remains an open one, and will vary among health-care systems.

Complex innovations in the field of health-related biotechnologies

Complex innovations such as those often found in the field of human health-related biotechnology may not only provide new opportunities to deliver better health outcomes, but also challenge current assessment techniques and decision making. For example, what are the disbenefits of a genetic test that impacts on the person's insurability, or of implicit assumptions about the value of increasing length of life in old age? How does society

value technologies that may be highly effective but targeted at very small patient groups and therefore costly?

Decision makers will increasingly require more evidence on all aspects of medical innovations, not just the clinical and economic effect but also ethical, social and macroeconomic characteristics, particularly as new health-related biotechnologies come on stream. The question is whether HTA can or should adapt its methodologies to capture these issues, or whether such evidence should come from other sources.

More empirical work is needed to open "the black box" of decision making, to see better how policy makers use the various tools and evidence available to them. However one problem is that decision makers may often find it impossible to make explicit their values and end points. For example, politicians who expose their desire to allow individual inequities in order to maximise general welfare may not find public support for such views. There is a need to explore possible policy frameworks in which these issues can be addressed, including the development of toolkits that can deliver relevant information to decision makers. The absence of a clear policy framework to deliver much needed information to decision makers creates greater uncertainty for innovators. The development of a policy framework could not only help decision makers maximise the benefits of biotechnologies in health-care systems, but also for reducing risk to innovators. Moreover, the lessons learned from such an exercise might be applicable to technologies that exhibit similar complex characteristics.

New biomedical advances have thrown into sharp relief the need for a better dialogue over the right questions to ask about technologies, and the right mechanisms - technical and political - for answering them. This in turn highlights the need for more appropriate training and development for assessors and decision makers. Nevertheless much closer links are needed between societal values, technology innovators and assessors.

From HTA and decision making to innovation

Over the course of the last century health technologies have contributed significantly to delivering better health outcomes. Developments in the science base, particularly in the life sciences, promise even more effective technologies for future health care provision as well as contribute to economic growth.

New innovations are importantly shaped by the demand conveyed by its purchasers. Increasingly, OECD countries are recognising the dynamics of medical innovation and have started to take account of the health-care system's role in encouraging significant innovation. Some policy frameworks are taking greater explicit account of the feedback signals from users to the R&D sector which often emphasised shortcomings in efficacy, safety, and ease of operation, but placed less emphasis on the need for value for money.

It is not clear whether these signals are always true reflections of the value placed on new innovation or whether they are distorted through various public and private actions[3]. In other words, the collective actions of health-care decision makers, as well as the actions of public policy makers, send signals to innovators. Yet such signals do not always provide the most appropriate and coherent indicators for the development of technologies in much needed areas.

3. HTA can play a role by highlighting areas of uncertainty, suggesting areas for further research, and outlining where changes might be needed to improve, for example, the cost-effectiveness of technology.

In principle, defining areas of health care priority could help guide the direction of innovation to deliver a better match between innovation and a society's health needs. However, there is only limited information on the practical steps that OECD member countries take to deliver such a match.

The analysis in this report shows that many OECD countries are employing HTA to deliver information to policy and decision makers to enable them to make more informed choices. However, many significant challenges remain in establishing a policy environment that sets the conditions to deliver the most effective and efficient technology to the right patients at the right time. Furthermore, challenges remain around the need for greater convergence between health priorities and innovation.

Annex 1

PARTICIPANTS IN THE EXPERT MEETINGS ON HEALTH-RELATED TECHNOLOGIES

Ms. Christiane AVELINE, Permanent Delegation of France to the OECD
Mr. Armando ALBERT, Centre d'Information et de Documentation Scientifique, Spain
Mr. José AMATE BLANCO, Agencia de Evaluacion de Tecnologias Sanitarias, Spain
Mr. Heinz BADER, Novartis Pharma AG, Switzerland
Ms. Alena BLAZKOVA**,** Ministry of Education, Youth and Sports, Czech Republic
Ms. Rosemary BOOTHMAN, Department of Health & Children, Ireland
Mr. Bengt BRORSSON**,** Swedish Council on Technology Assessment in Health Care (SBU)
Mrs. Helena BRUS, Merck & Co. Inc., United States
Ms. Milagros CASALS ARQUIMBAU, Permanent Delegation of Spain to the OECD
Ms. Aline CHABLOZ, Permanent Delegation of Switzerland to the OECD
Mr. Damian COBURN, Department of Health & Ageing, Australia
Ms. Jane COOK, Department of Health & Ageing, Australia
Ms Agnes CZIMBALMOS, National Institute of Health Promotion, Hungary
Mr. Andrew DILLON, National Institute for Clinical Excellence, United Kingdom
Mr. Luis DURAN, Mexican Institute of Social Security
Mr. Ralf EKEBOM, Ministry of Social Affairs and Health, Finland
Mrs. Anne-Florence FAY, CEDIT, France
Ms. Laurence FORT, MEDTRONIC Europe, Switzerland
Mr. Paal FRISVOLD, SPRL, Belgium
Ms. Maria GALLI, Istituto Superiore di Sanita, Italy
Mr. Felix GURTNER, Département fédéral de l'Intérieur, Switzerland
Mr. Esko HÄNNINEN, STAKES - National Research and Development, Finland
Mr. Ingo HÄRTEL, Federal Ministry of Health, Germany
Mr. Jan-Willem HARTGERINK, Ministry of Health, Welfare and Sport, Netherlands
Mr. Kwang-Lae HOE, Research Institute of Bioscience and Biotechnology, Korea
Ms Leena HOMMO, Ministry of Trade and Industry, Finland
Ms. Melissa HORWITZ, Permanent Delegation of the United States to the OECD
Mr. Jeroen HULLEMAN, Ministry of Health, Welfare and Sport, Netherlands
Mr. Yuichi IMANAKA, Kyoto University Graduate School of Medicine, Japan
Mr. Phil JACKSON, Ministry of Health and Long-Term Care, Canada
Ms. Anne JANIN, CHU St. Louis, France
Ms. Christine JESTIN, Ministère de la Santé, France
Mr. Richard JOHNSON, Arnold & Porter, United States
Ms. Susanna JONAS, Austrian Academy of Science, Austria
Mr. Thomas KOCH, Permanent Delegation of Switzerland to the OECD
Mr. Masato KUMAKI, Permanent Delegation of Japan to the OECD

Ms. Aline LAUTENBERG, EUCOMED, Belgium
Ms. Diane LUGSDIN**,** Health Canada
Mr. Zoltán MARCSEK, József Fodor National Public Health Centre, Hungary
Mr. Lars-Åke MARKÉ, Swedish Council on Technology Assessment
Mr. Pascal MEEUS**,** Organisation of Healthcare Establishments**,** Belgium
Ms. Teresa MORALES, Ministerio de Sanidad y Consumo, Spain
Mr. Juan Carlos MORENO, Permanent Delegation of Mexico to the OECD
Ms. Berit MØRLAND, Norwegian Center for Health Technology Assessment
Ms Lyndsay MOUNTFORD, European Commission
Mr. Richard MURRAY, Department of Health, United Kingdom
Mr. Akio NISHIMURA**,** National Institute of Public Health**,** Japan
Mr. Machel E.M. NUYTEN**,** Confederation of Netherlands Industry and Employers, VNO-NCW
Ms. Marie-Odile OTT, Direction de la Technologie, Ministère de la Recherche, France
Mr. Matti OIVUKKAMAKI, Ministry of Trade and Industry, Finland
Prof. José Luis PINTO, Universidad Pompeu Fabra de Barcelona, Spain
Ms. Nathalie POUTIGNAT**,** Agence Nationale d'Accréditation d'Evaluation en Santé, France
Ms. Nicole PRIMMER, Business and Industry Advisory Committee to the OECD (BIAC)
Mr. Alexandre QUINTANILHA, Universidade de Porto, Portugal
Mr. Luca Alessandro REMOTTI, FORMIT Foundation, Italy
Mr. Luc RIFFLET, Permanent Delegation of France to the OECD
Mr. John ROBINSON, Permanent Delegation of the United States to the OECD
Ms. Hanni ROSENBAUM, Business and Industry Advisory Committee to the OECD (BIAC)
Mr. Manuel RUIZ DE CHAVEZ GUERRERO, Fondacion Mexicana para la Salud (Mexican Health Foundation)
Mr. Alric RUTHER, German Agency for Health Technology Assessment
Ms. Jill SANDERS, Canadian Coordinating Office for Health Technology Assessment (CCOHTA)
Mr. Markus SIEBERT, EUCOMED, Belgium
Mr. Alain SOMMER, USINOR INDUSTEEL, France
Mr. Keiji TAKEBAYASHI, Permanent Delegation of Japan to the OECD
Ms. Tania TESCHKE, Permanent Delegation of the United States to the OECD
Ms. Bernie TOWLER, Department of Health & Ageing, Australia
Ms. Adriana VELAZQUEZ-BERUMEN**,** Ministry of Health, Mexico
Mr. Carl VINCENT, Department of Health, United Kingdom
Ms. Salome VON GREYERZ, Office Fédéral des Assurances Sociales, Switzerland

Annex 2

"SEIZING THE OPPORTUNITIES AND MEETING THE CHALLENGES OF NEW AND EMERGING HEALTH TECHNOLOGIES": WORKSHOP PROGRAMME

The Hague, Netherlands

Monday, 27 October 2003

Welcoming Address: Roel Bekker, Secretary General, Ministry for Health, Welfare and Sport, Netherlands

Workshop Goals: Iain Gillespie, Head of the Biotechnology Unit, OECD

Session 1. Health Technology Assessment and Decision-Making: The State Of Knowledge

Chair	Berit Morland, Norwegian Center for Health Technology Assessment, Norway
Presenters	Finn Borlum Kristensen, Danish Centre for Evaluation and HTA, Denmark Pascale Lehoux, Université de Montréal, Canada

Session 2. The Health Technology Decision-Making Infrastructure

Chair	Andrew Dillon, National Institute for Clinical Excellence, United Kingdom
Presenters	Egon Jonsson, WHO Regional Office for Europe, Denmark Kees van Gool, Biotechnology Unit, OECD
Panel	Egon Jonsson, WHO Regional Office for Europe, Denmark Akio Nishimura, National Institute of Public Health, Japan Frans Rutten, Institute for Medical Technology Assessment, Netherlands

Session 3. Translating Evidence into Policy and Policy into Practice

Chair	Jill Sanders, Canadian Co-ordinating Office for Health Technology Assessment, Canada
Presenters	Andrew Dillon, National Institute for Clinical Excellence, United Kingdom Albert Boer, College Care Insurance Board, Netherlands Kees van Gool, Biotechnology Unit, OECD
Panel	Andrew Dillon, National Institute for Clinical Excellence, United Kingdom Alric Ruther, German Agency for Health Technology Assessment, Germany Albert Boer, College Care Insurance Board, Netherlands Bengt Brorsson, The Swedish Council on Technology Assessment in Health Care, Sweden

Session 4 (Part 1). Broader Challenges to HTA and Policy

Chair	Gabriël Ten Velden, Health Council, Netherlands
Presenters	Luis Duran, Mexican Institute of Social Security, Mexico John Bridges, Case Western Reserve University, United States Kees van Gool, Biotechnology Unit, OECD
Pane	Luis Duran, Mexican Institute of Social Security, Mexico Ingo Haertel, Federal Ministry of Health, Germany Damian Coburn, Department of Health and Ageing, Australia John Bridges, Case Western Reserve University, United States

Tuesday, 28 October 2003

Session 4 (Part 2). Broader Challenges to HTA and Policy

Chair	Luis Duran, Mexican Institute of Social Security, Mexico
Presenters	Ingo Haertel, Federal Ministry of Health, Germany Damian Coburn, Department of Health and Ageing, Australia
Panel	Gabriël Ten Velden, Health Council, Netherlands Ingo Haertel, Federal Ministry of Health, Germany Damian Coburn, Department of Health and Ageing, Australia

Session 5. Pharmaceuticals: Decision Making and Assessment

Chair	Damian Coburn, Department of Health and Ageing, Australia
Presenters	Frans Rutten, Institute for Medical Technology Assessment, Netherlands Jill Sanders, Canadian Coordinating Office for Health Technology Assessment, Canada Albert Wertheimer, Centre for Pharmaceutical Health Services Research, United States Claus Moldrup, The Danish University of Pharmaceutical Sciences, Denmark
Panel	Frans Rutten, Institute for Medical Technology Assessment, Netherlands Jill Sanders, Canadian coordinating Office for Health Technology Assessment, Canada Claus Moldrup, The Danish University of Pharmaceutical Sciences, Denmark Albert Wertheimer, Centre for Pharmaceutical Health Services Research, United States

Session 6. Workshop Conclusions

Chair	Iain Gillespie, Head of the Biotechnology Unit, OECD
Rapporteur	John Gabbay, Wessex Institute for Health Research and Development, United Kingdom

Annex 3

SUMMARY OF SURVEY QUESTIONS

For each of the five case study technologies, participants in survey countries were invited to respond to the following set of questions?

Part A: Health care system and technology background

- Where in the health care system is the technology provided?
- How do patients gain access to the technology?
- What policy mechanisms are in place to manage diffusion and use of technology?
- What provider payment mechanisms are in place, related to the use of the technology?
- How was/is the technology financed?
- Timeline of significant events of assessment and decision making for the technology.

Part B: Health technology assessment and evidence on the technology

- Has a HTA been produced in your country for this technology?
- How long did the HTA take to complete and what were the costs?
- What information did the HTA contain and what was the source of that information?
- Were patient preferences included?
- What were the main recommendations of the HTA?
- Did HTA confirm or contradict prior expectations?
- What was the envisioned role of and reason for the HTA?
- Who conducted and funded the HTA?
- Who was the primary audience and how was the HTA disseminated?
- Were multiple HTAs on this technology undertaken in your country?
- What are the biggest challenges for HTA in your country?

Part C: How was the decision to (or not to) integrate the technology into the health care system made?

- Where was the decision made?
- What instigated the need for a decision?
- What decision can decision-makers make, and what decision did they choose?
- Who was involved in the decision making process, and who made the decision?
- Did the process comply with the four conditions of "reasonable" decision-making?
- Are decisions accepted by stakeholders?
- Was HTA a formal requirement of the decision-making process?
- Were foreign or domestic HTA available and used in the decision?
- If foreign, was there an attempt to place the results in a national or local context?
- How important were specific types of evidence in the decision?
- Was HTA suited to decision-makers needs? In what ways could HTA have been more useful to the decision-making process?
- Has HTA met, exceeded or disappointed your expectations?

Part D: Decision implementation

- How was the decision translated into policy?
- How was the decision translated into practice?
- Have there been any evaluations on the effectiveness of mechanisms aimed at influencing uptake of the technology through providers or citizens/patients?
- What features of the health care system assist in transferring policy into practice?
- What factors are regarded as the main health system barriers in translating policy into practice?
- If the decision was to implement, was it backed with new funding?

Part E: Outcomes

- What, if any, monitoring occurs to determine whether recommendations are implemented?
- Please provide details, including references, of any existing evidence on the following measures:
  - What was the variation between actual practice and recommended practice over time?
  - What was practice before and after decision (post-decision surveillance) over time?
  - What is the level of technology diffusion (and rate of) over time?
  - Was there a change in health outcomes over time?
- Does the health system collect data on the four measures listed above?

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The OECD Health Project

Health Technology and Decision Making

Health technology has the tremendous potential to change our understanding of disease, transform the delivery of health-care services, and improve health outcomes. But using such technology comes at a price. Decisions about whether to purchase and use new technology should be based on high-quality evidence on its impact on health care and health outcomes.

OECD countries face the challenge of aligning health-care decision making with the best available evidence. While many countries have invested in the production of health technology assessment, evidence about its use in decision making is limited.

Health Technology and Decision Making analyses the barriers to, and facilitators of, evidence-based decision making in OECD health-care systems. It examines how countries can successfully manage the opportunities and challenges arising from health-related technology by optimising decision-making processes, recognising the value of innovation, dealing with uncertainty, and producing and co-ordinating health technology assessment. The book also considers the capacity of health systems to respond to the particular challenges of fast-developing health-related biotechnologies.

See the OECD website on Health at: ***www.oecd.org/health***.

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