The systematic use of cost-effectiveness criteria to inform reviews of publicly funded benefits packages

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Introduction

1. Technological advances, demographic changes and increasing expectations contribute to growing pressure on limited health budgets. Health systems are striving to provide accessible health care of high quality that is responsive, affordable and financially sustainable, but limited resources have meant that there is a growing tension between the possibilities offered by medical interventions and the ability (and willingness) of public payers[1] to provide unlimited access to all health services [1].

2. Policy makers need to make decisions about the use of public funds and countries are increasingly engaged in developing formal systems to support this process, such as the use of evidence-based medicine and the application of cost-effectiveness analysis (CEA) of health interventions, for example through the implementation of formal systems of health technology assessment (HTA).

3. Here we review the systematic use of CEA to inform decision-making about publicly-funded services and interventions in the health sector. The review is in two parts. Part 1 begins with a brief overview of HTA/CEA systems in place in 11 countries before moving on to exploring in greater detail specific organisational, structural and technical aspects of CEA programmes in selected countries that use CEA to evaluate *existing* interventions. Part 2 expands the synthesis presented in Part 1, including a rapid review of the international experience of approaches to reviewing and/or redefining benefits packages and identifying potential factors that enable or hinder formal decisions to exclude services from the publicly-funded benefits package.

4. This rapid review has been informed by several sources, including (a) information provided by the members of the International Healthcare Comparisons network, (b) specific country informants providing expertise on CEA programmes and methodologies and (c) an iterative document and literature search using academic databases, world wide web search engines and specific government and agency websites. As published information on the use of CEA to assess existing interventions is limited, this assignment mainly draws on information provided by experts in the network and beyond.[2]

[1] In this review 'public payers' refers to government authorities involved in allocating tax-based revenues and to social health insurance bodies, both at national and/or regional level (as applicable).
[2] The contributions of experts external to the IHC network are indicated.

PART 1

Overview

5. HTA "is a form of policy research that systematically examines short- and long-term consequences of the application of a health technology, a set of related technologies, or an issue related to technology" [2]. CEA and its variants cost-utility analysis (CUA) or cost-minimisation analysis (CMA) are frequently used in HTA, although CEA programmes are not necessarily exclusively undertaken by organisations or institutes that are formally recognised as the national/regional HTA agency. The scope of CEA programmes and organisational arrangements thus varies widely between countries. Table 1 shows that all of the 11 countries included in this overview have implemented a formal HTA system. Of these, most countries use CEA to evaluate new technologies, with a large emphasis on pharmaceuticals, but only a few countries also make systematic use of CEA to evaluate existing interventions to inform decision making.[3]

Table 1: Systematic use of health technology assessment (HTA) and cost-effectiveness analysis (CEA) in selected countries

	AUS	DEN	FIN	FRA	GER	ITA	NET	NOR	NZ	SPA	SWE
HTA	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓
CEA of new intervenet ions	✓	x*	✓	x*	as of April 07	x*	✓	✓	✓	x*	✓
CEA of existing interventi ons	✓	x	x	x	x	initiat ed in Feb 07	idea stage	idea stage	✓	x	✓

Note: 'Interventions' primarily denote pharmaceuticals and other health technologies where applicable.

*CEA may be used occasionally or in support of an application for the inclusion of drugs and technologies (e.g. by pharmaceutical suppliers) in the public reimbursement schedule but is not used systematically as part of a formal programme and is not an explicit criterion for decision making.

6. CEA is undertaken by various organisations, including government or arm's length bodies, departments within the Ministry of Health, universities and other research organisations and private companies such as suppliers of pharmaceuticals or medical devices. Here we focus on formal, government-run or commissioned programmes that systematically conduct CEA.

7. The scope of HTA/CEA has gradually expanded in many countries and increasingly covers not only pharmaceuticals and medical and surgical interventions but also health policies (for example, those related to organising and financing health care)

[3] Throughout the document the term 'interventions' denotes all medical and related health care treatments, including pharmaceuticals and procedures. The terms 'health care technologies' and 'health services' are used interchangeably.

[3]. The main focus of CEA is the evaluation of new and expensive technologies. However, there is a growing awareness in some countries of the need to assess the cost effectiveness of existing interventions (i.e. pharmaceuticals and other treatments that are already funded by the public system) as a way of reviewing and possibly redefining the scope of the publicly-funded benefits package. Here we focus on *systematic* approaches to assessing the cost effectiveness of existing interventions.

Systematic assessment of the cost effectiveness of existing interventions

8. Among the countries we reviewed, only a small proportion have established formal programmes to evaluate the cost effectiveness of existing interventions. Of these, only Australia, New Zealand and Sweden use CEA to inform decisions about the scope of the publicly-funded benefits package (which may involve de-listing or disinvestment). However, there appears to be growing interest in establishing similar programmes elsewhere and a further three countries have either recently initiated a programme (Italy in February 2007) or are currently discussing the possibility of doing so (the Netherlands and Norway).

9. CEA of existing interventions appears to form only a minor component of larger CEA programmes (with the possible exception of Sweden). As noted above, most formal programmes are primarily concerned with the evaluation of new interventions in the context of post-licensing procedures and to determine eligibility for public funding. Existing interventions nevertheless play a key role in most CEAs as they usually provide the comparator for CEA of new interventions (i.e. new interventions are compared to current practice). Where the new intervention is found to be more cost-effective, the intervention it supersedes may eventually be replaced (e.g. in New Zealand). However, such an approach may be seen as a by-product of CEA of new interventions and it is not clear whether it should be considered as a systematic approach to CEA of existing interventions.

10. Organisations involved in CEA programmes are usually arm's length bodies with a national remit (Table 2). The nature and scope of their mandate varies. The literature distinguishes between *assessment* and *appraisal*; assessment is the science that underlies HTA (i.e. the HTA study), while appraisal refers to the process by which the science is considered at the policy-making level [4]. A key difference among the organisations reviewed here is whether the remit of the organisation is limited to *assessment*, with decision-making left to other actors, or whether it incorporates appraisal and is therefore involved in decision-making. Table 3 shows that, in the countries under review, organisations assessing pharmaceutical products have the authority to make binding decisions (i.e. regulatory powers), while those involved in assessing medical procedures do not.

Table 2: National organisations involved in CEA of existing interventions

Country	Institutions
Australia	The Pharmaceutical Benefits Advisory Board (PBAC) and the Medical Services Advisory Committee (MSAC) conduct CEA of new and existing interventions (i.e. of drugs and procedures respectively).
Italy	In February 2007 the Italian government initiated a new programme (the National System for the Review and Control of the Health System) aimed at revising the national benefits package which has to be offered by the regions (free of charge or subsidised).
Netherlands	Following the health insurance reform of January 2006 the Dutch government is considering introducing a policy which would widen the scope of CEA conducted by the Health Care Insurance Board (CVZ), an arm's length body responsible for regulating social health insurance.
New Zealand	The Pharmaceutical Management Agency (Pharmac) undertakes, among other things, CUA of pharmaceuticals considered for public subsidy. Most analyses are carried out on new drugs but may also involve CUA of existing drugs. There is no such programme for other interventions.
Sweden	The Pharmaceutical Benefits Board (LFN) uses CEA to determine whether drugs should be publicly reimbursed. The National Board of Health and Welfare (NBHW) develops clinical guidelines which take account of CEA. The Swedish Council on Technology Assessment in Health Care (SBU) develops and disseminates HTA information, including CEA.

Table 3: Remits of organisations involved in CEA programmes

Country	Assessment only	Assessment and appraisal
Australia	**MSAC** provides information on cost effectiveness of medical services; analyses are advisory to the Minister of Health.	In the pharmaceutical sector, **PBAC** has the authority to decide about the exclusion of drugs from public subsidy. Its decisions are binding for all states and territories, unless they are overturned by national legislation.
New Zealand		The **Pharmac** board decides on whether a drug is eligible for public subsidy. Pharmac's decision is binding for all District Health Boards across New Zealand.
Sweden	The **NBHW** mainly provides guidelines aimed at supporting priority-setting at the clinical level. **SBU** provides information on HTA; CEA may play a minor role.	**LFN** decisions on the eligibility of drugs for public funding are binding.

11. Technical approaches to the application of CEA do not seem to differ greatly between countries (e.g. the type of data and the quality of evidence used in economic evaluations). Most CEA uses cost per QALY as a conceptual base (Australia, New Zealand, Sweden) and, conceptually, there seem to be no systematic differences between CEA of new technologies and CEA of existing interventions. However, there may be differences with regard to the selection of interventions for CEA, which determines the nature of both the data and the analysis to be undertaken (e.g. pharmaceutical companies may exclusively provide documentation of analyses for new drug applications, whereas reviews of existing drugs may involve additional modelling and analyses, not previously provided by a third party; see the case study on Sweden).

12. The role of CEA in decision-making: Although the extent of experience reviewed here is limited, it seems that information on cost effectiveness constitutes only one criterion for decision-making. The most commonly-used criteria are safety and effectiveness but other criteria (including political considerations) may also play an important role. However, it is not always evident how different criteria influence the decision-making process.

13. Use of 'ethical weights': None of the three countries considered in this review uses ethical weights when carrying out economic analysis to inform funding decisions. Rather, decision makers are usually provided with a set of criteria on which the decision to fund (or de-list) interventions should be based. The level of detail and explicitness of the criteria used varies between countries (Table 4). Criteria tend to be worded in general terms and analysts may be reluctant to assign specific weights to individual criteria, suggesting that each case has to be considered separately. This may reflect a potential trade-off between the desire to define explicit criteria and the need for flexibility and political sensitivity in decision making.

Table 4: Role of CEA as a funding criterion

Country	Role
Australia	PBAC's decisions are based on safety, comparative clinical effectiveness and cost effectiveness. PBAC also has a formal policy on the use of the 'rule of rescue'.
New Zealand	Cost effectiveness is one of nine explicit principles on which Pharmac bases its decisions, including clinical risks and benefits, alternative treatment options, the impact of the decision on public funding, other government priorities and the health needs of the entire population as well as the specific health needs of Maori and Pacific people.
Sweden	Decision-making in health care in Sweden is based on three principles, which also guide priority-setting and reimbursement decisions. These principles are human dignity, the principle of need and solidarity, as well as cost effectiveness [5].

14. Marginal cost effectiveness for different patient groups: Programmes across countries usually use sub-group analysis to determine the cost effectiveness of an intervention for specific patient groups, usually those who are likely to benefit most from the treatment. However, sub-group analysis relies on the availability of specific

data and evidence from clinical trials. As a result of sub-group analysis, funding decisions tend to specify treatment for certain indications (e.g. severity of the condition; co-morbidities; other risk factors).

PART 2

Defining the benefits package: some international experiences

15. During the 1990s, policy-makers in a number of countries have supported the formal review of the health care benefits packages in their constituency with a view to defining explicitly which health services would be publicly funded. The most prominent example of this form of explicit rationing first took place in the state of Oregon in the United States in 1989 [6], with countries such as Israel and New Zealand following suit.[4] Although Israel is not part of the International Healthcare Comparisons network, we include a brief overview of its experience of priority setting as it highlights some controversial aspects of explicit rationing.

16. New Zealand (1992): In 1992 the incoming centre-right government established a National Advisory Committee on Core Health and Disability Support Services ('Core Services Committee', CSC) to compile a list of essential (and cost effective) health services to be publicly reimbursed, along the lines of the Oregon list. However, following extensive public and professional consultation the committee eventually rejected the idea of defining a clear-cut benefits package in 1994. Instead, it recommended the development of clinical guidelines to guide treatment decisions at the clinical level. Nevertheless, controversy has surrounded cases in which treatment has been denied (on the basis of the guidelines). A more detailed review of the New Zealand experience is provided in the annex [8].

17. Israel (1997): In 1997 Israel established a formal priority-setting process for assessing services to be added to the benefits package stipulated by the 1995 National Health Insurance Law [9]. A public committee representing health plans, the Ministry of Health, the Ministry of Finance, the Israel Medical Association, health economists and health policy and public actors from outside the health system recommends which new technologies (from among those already identified and economically evaluated by the Ministry of Health) should be adopted and final decisions are made by the Minister of Health. However, proposals to remove services (e.g. IVF) by health plans and others have usually been strongly opposed by the public and as of 2003 none had been formally considered by the public committee or accepted.

18. Sweden (2002): The Pharmaceutical Benefits Board (LFN) established in October 2002 sets prices for outpatient prescription drugs and determines their eligibility for public reimbursement. LFN also reviews drugs that were approved for public reimbursement before 2002. This review includes some 2000 drugs and assessments are undertaken by therapeutic group. Since 2003, LFN has completed

[4] 'Rationing' health care has been defined by Klein et al. as involving "the denial or dilution of something that is potentially beneficial to the patient: he or she is getting less in way of treatment than might be thought desirable in a world with unlimited resources" [7: 6].

a review of the reimbursement status of two groups: medicines used for the treatment of migraine and antacids. Forthcoming treatment areas that are subject to review are high blood pressure, asthma, depression and high cholesterol levels. A total of 49 groups of pharmaceuticals have been identified and prioritised for review, with selection criteria based on budgetary impact. It is noteworthy that the approach chosen in Sweden is different from the experience of New Zealand (i.e. the attempt to define 'core services') and Israel, both tending to propose the exclusion of entire treatment areas (e.g. IVF in Israel or dialysis for end stage renal failure in New Zealand). In contrast the Swedish approach involves reviewing all pharmaceuticals within a treatment area, limiting access to those that are the least cost effective (e.g. by defining criteria for treatment) while permitting public reimbursement of alternative treatment options.

19. The challenge of defining an explicit benefits package has also been illustrated by a recent review of the use the formal HTA processes to inform decision making in England and Wales, France, the Netherlands and Sweden. The review suggests that where evaluations of existing interventions have been used, these tend to take the form of clinical guidelines, allowing leeway for individual decision made by clinicians [10]. However the review also found that guidelines rarely incorporate information on cost effectiveness. Rutten (2004) noted that where systematic assessment does have an influence, it may be more in the form of limiting the use of a technology to a particular sub-group rather than rejecting it outright [10].

Areas for further consideration

20. In light of limited public health budgets and the potential for savings to be generated from the removal of public funding for health interventions considered to be not cost effective, it is noteworthy that (a) so few countries appear to have established programmes to assess systematically existing interventions against their (cost) effectiveness and (b) even countries that have established relevant programmes or systems have as yet presented few examples involving the explicit exclusion of interventions from public funding following evaluation.

Against this background a number of key questions about challenges and potential obstacles to reviewing benefits packages on the basis of cost effectiveness emerge:

21. Political context: As noted above, rationing decisions tend to be controversial, especially if they aim to be explicit and transparent. Decisions about the explicit exclusion of interventions from the benefits package should be informed by evidence, yet they also involve values. Thus, de-listing decisions are unlikely to be amenable to technical solutions alone. Consequently, any attempt to balance population and individual needs is more than simply a matter for analysts (however well intentioned they may be). Effective programmes will require considerable political will [11] and may be more successful if they account, from the outset, for policy processes (and, possibly, for legislative and judicial issues). The process may be more likely to be successful if the organisation involved is independent of government or at arm's length from the political process.

22. Resources: In the countries reviewed here the organisation responsible for assessment of existing interventions also evaluates new technologies (with the possible exception of Italy). Agencies involved in HTA and CEA often have to operate within the constraints of limited financial and human resources and thus reviews may be biased towards new interventions, assessment of which tends to be more urgently demanded (e.g. by pharmaceutical companies, patients and policy-makers). There may therefore be a case for devoting separate resources to facilitate reviews of existing interventions (e.g. through earmarked funding).

23. Transparency and public involvement: Some countries have clearly defined principles and criteria on which funding decisions are based and make these available publicly (e.g. New Zealand, Sweden). Yet even where criteria have been made explicit, they tend to be rather general, possibly to allow for a degree of flexibility in decision making and a weighing of risks and benefits. Transparency in decision making may increase the legitimacy of the evaluation process, although not necessarily the acceptance of its outcome (e.g. the withdrawal of funding). It may also inadvertently strengthen the position of interest groups [12]. Acceptance may be strengthened by systematically involving patients and the wider public. At the same time, processes that rely heavily on public consultation may be more vulnerable to opposition and, potentially, interest group capture. There is a perception that some countries with a history of explicit rationing and related debates (e.g. New Zealand [8]) may have experienced growing awareness of the need to ensure that publicly-funded services provide 'value for money', including health care. It is uncertain, however, to what extent awareness of the underlying problem translates into acceptance of controversial decisions.

24. Ethical concerns: Even though the need for priority setting may in general be understood and accepted, there may be unease about the process, particularly where economic evaluation is involved. Some may consider it unethical to place a monetary value on life or quality of life and clinicians may feel they have a duty to treat all cases [13]. Clinicians (and members of the public) may also feel that decisions to exclude interventions are more likely to be acceptable if they are based on criteria such as safety and effectiveness rather than cost effectiveness.

Part I

Australia

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Formal status of organisations and programmes performing CEA of existing interventions

Several programmes and organisations in Australia undertake health technology assessment (HTA). The assessment of cost-effectiveness (CEA)[5] for goods and services included in the national benefit package is undertaken by two national-level bodies, the Pharmaceutical Benefits Advisory Committee (PBAC) and the Medical Services Advisory Committee (MSAC), with CEA carried out for pharmaceuticals and medical services. Table 1 sets out the national agencies or committees using HTA with responsibility for the various technologies, and the assessment criteria that they use.

Table 1 National Agencies/Committees involved in HTA and key assessment criteria

Agency or committee	*Technologies assessed*	*Safety*	*Clinical efficacy*	*Clinical effectiveness*	*Cost effectiveness*
TGA	Medicines and devices [(a)]	√	√		
PBAC	Pharmaceutical products			√	√
MSAC	Procedures, devices and equipment	√		√	√
ASERNIP-S	Surgical procedures	√	√		
PDC	Devices and prostheses		√		
ATAGI	Vaccines [(b)]	√	√		

Notes: (a) TGA also covers blood, tissue and cellular therapies; (b) PBAC assumed responsibility for assessing cost effectiveness of new vaccines in 2006 [14: 185].

[5] The Productivity Commission defined cost-effectiveness analysis (CEA) as providing "a method of evaluating the relative healthcare outcomes of various technologies, without placing monetary values on benefits. The outcomes of a particular intervention are expressed as the cost per unit of benefit. Benefits can be expressed as lives saved, illnesses diagnosed, repeat procedures avoided or quality-adjusted life-years (QALYs) among others." [14: 168]

The Pharmaceutical Benefits Advisory Committee (PBAC) was established in 1954 as an independent statutory body under the *National Health Act 1953 (Cth)* to advise the national Minister for Health, and its negative recommendations have special legislative protections. Membership is by Ministerial appointment for a fixed term. Cost effectiveness analysis was made mandatory for new pharmaceuticals after 1987 following amendments to the *National Health Act,* requiring PBAC to consider the effectiveness and cost of a drug proposed for PBAC listing compared to other therapies or to no therapy.

Medical services are considered by the Medical Services Advisory Committee (MSAC) established in 1998, which is solely an advisory committee to the Minister for Health. Membership is by Ministerial appointment for a fixed term.

Several other bodies are involved in HTA, but to a lesser extent in cost-effectiveness analysis:

HealthPACT (Health Policy Advisory Committee on Technology) performs a horizon-scanning role for State government health departments, and shares secretariat and other functions with MSAC. Funded through the Australian Health Ministers' Advisory Committee (AHMAC), a committee of senior public servants from the national government and the States and Territories, HealthPACT also includes the National Horizon Scanning Unit that alerts the various health jurisdictions of Australia and New Zealand and responds to requests for advice on new and emerging technologies that may impact on their public health care systems within a three-year horizon.

AHMAC has delegated to MSAC a role in advising on 'super-specialty' services. These are generally services where the volume of cases is not sufficient to justify more than one or two units in the country. Historically, these have been transplant units. The 'super-specialty' designation ensures that all States contribute to funding of such units thus guaranteeing access for residents of all states.

The Therapeutic Goods Administration (TGA) within the Australian Government Department of Health and Ageing is responsible for the safety and efficacy of new therapeutic goods but is not required to assess their cost effectiveness. The *Therapeutic Goods Act 1989 (Cth)* defines a therapeutic good as anything used for the prevention, diagnosis or treatment of disease and other bodily conditions, so that the TGA is required to assess drugs, medical devices, blood, tissues and cellular therapies. It regulates the overall supply through (i) pre-market evaluation, (ii) licensing of manufacturers, and (iii) post-market surveillance. High risk products are evaluated for quality, safety and efficacy, and if approved, are placed on the Australian Register of Therapeutic Goods as ‘registered’ products; those assessed at lower risk are evaluated only for quality and safety and if approved are included on the ARTG as ‘listed’ products. As at 30 June 2004, there were about 10,500 registered medicines and around 16,600 listed medicines on the ARTG [14: 186].

The Prostheses and Devices Committee (PDC) was established in 2004 to advise and recommend to the national Minister of Health on the listing of new prostheses on the Prostheses Schedule and the setting of benefit levels that private health insurers need to cover for their members. It currently lists about 9000 items [14: 192].

The Australian Technical Advisory Group on Immunisation (ATAGI) was established in 1997 to advise the Minister on technical and scientific elements of the National Immunisation Program. It considers vaccines likely to be approved for use in Australia and liaises with the TGA and the Department of Health and Ageing on matters regarding the availability, safety and clinical effectiveness of vaccines. Its role in assessing the cost effectiveness of new vaccines was transferred to PBAC in 2006 in order to strengthen CEA recommendations [14: 191].

State Governments are developing technology assessment capabilities and processes to regulate the introduction of new technologies into their public hospitals (see below for a summary of jurisdiction roles). These processes usually rely on submissions prepared by public hospital physicians, and may or may not include consideration of cost-effectiveness. Some State Governments have established advisory committees and working groups to assess requests to use new medicines or other medical technologies in hospital settings. For example, the Victorian Policy Advisory Committee on Technology (VPACT) was set up in 2004 to promote a systematic HTA approach, and uses a variety of approaches including horizon scanning, assessment and monitoring [14: 193]. The Victorian Medicines Advisory Committee (VMAC) was established to advise on the safe, efficient and effective use of medicines within Victoria. Some Victorian regional health services have also established internal HTA committees to oversee the introduction of new medical procedures, for example at Bayside Health and at Southern Health [14: 194]. Similar mechanisms exist in Queensland, Western Australia and South Australia. These committees commonly consider applications for high price and/or high volume drugs, devices and procedures.

The national government provides direct funding for only one non-government HTA organisation, the Australian Safety and Efficacy Register of New Interventional Procedures-Surgical (ASERNIP-S). This organisation was created under the sponsorship of the Royal Australasian College of Surgeons (RACS) to develop national capacity in the assessment of surgical procedures in a large number of specialty areas. It also conducts horizon scanning for new and emerging surgical techniques. Once systematic reviews of selected new surgical procedures are endorsed by the RACS Council, they are disseminated among the relevant surgical sub-specialty organisations.

A number of other hospital or university-based HTA organisations are funded through medical research funding (e.g. the Screening and Diagnostic Test Evaluation Program [STEP]), or through contracts with MSAC and PBAC (e.g. Adelaide Health Technology Assessment, Monash Centre for Health Economics), or through a combination of research funds and contracts (*eg*, the National Health and Medical Research Council's [NHMRC] Clinical Trials Centre).

The private sector also has developed some capacity to undertake cost-effectiveness analysis. Private HTA firms contract with pharmaceutical companies to prepare submissions to PBAC, and with device manufacturers to prepare submissions to MSAC. Some private HTA firms (*eg*, M-TAG) also do contracted work for MSAC. Private health insurance funds cover a range of hospital and medical services and some pharmaceuticals, and although they mainly rely on government HTA processes, they may undertake in-house assessments of new drugs or of particular medical services or devices [14: 194].

Remit of organisations

Mandates of the organisations are as outlined above.

National legislation (Section 101(3) of the *National Health Act)* requires that when considering a proposal for listing with the Pharmaceuticals Benefits Scheme, PBAC take into account information on the comparative clinical effectiveness, safety and cost effectiveness of the new product. It is not precluded from also taking other factors into account, such as clinical need for the drug. Economic analyses are conducted by the PBAC Economics subcommittee upon whose advice PBAC decides whether a new drug represents "value for money".

Funding sources

Funding of the various HTA organisations is considered above; government funding arrangements as noted above are all derived from taxes, except for privately-financed HTA.

Use of recommendations to determine the nature and scope of the benefits package

As noted earlier, MSAC is an advisory committee to the Minister for Health and positive and negative recommendations are solely advisory, with all decisions resting with the Minister, and in some cases, the Cabinet.

Similarly, positive PBAC recommendations are purely advisory to the Minister for Health. However, while a positive recommendation by PBAC does not ensure listing, a recommendation *not* to list a drug requires legislative (not just Ministerial) intervention to be overturned. The Health Minister and Parliament thus may reject an affirmative PBAC recommendation to list a new drug or to amend its coverage, but they may not add a new drug to the PBS that has not been endorsed by PBAC. For example, the PBAC recommendation to list Viagra was rejected by the Australian Government in 2002 because of costs concerns [15]. While the PBAC role is advisory, it is a statutory authority and holds substantial power over the pharmaceuticals benefits scheme. Sponsors of a new product that receives a negative recommendation can resubmit later, usually with new data or a lower price. Appeals against the PBAC process (but not the decision) are permitted under the *Administrative Decisions (Judicial Review) Act 1977* (Cth). Delisting of drugs or services is not routine, and likely to generate controversy. The Minister has recently created a review mechanism to deal with sponsors who wish to appeal negative PBAC recommendations.

In addition, a new review mechanism for PBAC recommendations is being implemented under the Free Trade Agreement (FTA) signed by Australia and the US in 2004. Price determination is not part of the formal HTA process but PBAC listing recommendations are forwarded to the Pharmaceuticals Benefits Pricing Authority for price-setting purposes. It considers several factors in using a reference pricing system whereby the Australian Government pays a subsidy equal to the lowest price drug (the reference price) in the relevant group of drugs.

Organisations responsible for decision-making on the nature and scope of the benefits package

The full range of participants in decision-making is outlined above.

Responsibility for health care in Australia is divided between the national (Australian) Government and the eight States and Territories. The Australian Government subsidises medical services (GPs and specialists) through Medicare, and out-of-hospital pharmaceuticals through the Pharmaceutical Benefits Scheme. It regulates and subsidises the private health insurance industry. Recommendations on the inclusion of services and drugs considered sufficiently cost-effective in this subsidised benefits package are made to the Australian Government Minister by MSAC and PBAC, respectively. The Australian Government subsidises but does not manage or make decisions regarding the State public hospital systems. Individual health insurers decide which allied health interventions will be covered.

State Governments fund (along with funds from the Australian Government through the Australian Health Care Agreements) and operate systems of public hospitals, including the costs of all in-hospital medical services, allied-health services, and pharmaceuticals. Limited numbers of community-based allied health professionals are also employed by State Governments. The States also regulate private hospitals.

Scale of organisation's ambition

Both national committees are submission-based, so the scope and scale of their work are externally determined. Occasionally the Australian Government Department of Health and Aging refers medical services to MSAC for consideration of changes to restrictions (essentially, delisting services for some indications), but this forms only a small part of the work program.

The Pharmaceutical Benefits Scheme (PBS) is a major government program with expenditure of around $5.7 billion in 2003-04, accounting for 11.6% of total recurrent health expenditure [16]. The number of drugs listed on the PBS has grown from 139 in 1948 to about 650 in 2004 (in 1600 dosage forms). PBAC has so far evaluated nearly half of the PBS-listed pharmaceuticals for cost-effectiveness (46% in 2003/4 compared to 4% in 1992/93) and aims to gradually extend a cost-effectiveness assessment that will involve de-listing some older drugs. Of those drugs that had been assessed only few have been re-assessed after listing [14: 188].

Medical services (services provided by medical practitioners primarily on a fee-for-service basis) accounted for 17.1% of total recurrent health expenditure [17]. The Medicare Benefits Schedule (MBS) lists and describes the medical and diagnostic services for which a Medicare benefit is payable by the Australian Government. The MBS applies to medical services in the community and also to medical services provided to private patients in both public and private hospitals. In 2004, there were more than 4500 individual items listed on the MBS and supplementary schedules, including many commonly used clinical medical procedures that have however never been subjected to a cost effectiveness analysis [14: 189]. In June 2004 MSAC had completed around 70 evaluations of new services, of which 52% were therapies, 23% related to diagnostic imaging, 13% to other diagnostic tests and 12% to diagnostic pathology [14: 190].

The States together determine HealthPACT's budget and work programme in horizon scanning. Individual public hospitals undertake HTA as the basis for a submission to State government, usually to allow introduction of a novel or state-wide specialty service outside normal hospital funding arrangements.

Volume of financial resources dedicated to the organisations

Funding for PBAC, MSAC and HealthPACT health technology assessment activities is not separately identified in government health budgets. Political pressure on these bodies to process submissions in a commercially-timely fashion ensures substantial funding to perform their HTA roles.

University and hospital-based HTA organisations are dependent on grants and contracts to sustain a work programme, and funding is variable and uncertain.

Human resource capacity dedicated to the organisations

Not possible to quantify (see above).

Selection process of interventions for CEA

All PBAC and MSAC recommendations must include a cost-effectiveness analysis, or at least a cost-minimisation analysis, in support. Priorities are determined on the basis of applications for subsidy, nearly all of which are formally considered; drugs and devices not approved by the Therapeutic Goods Administration are the major exception. The restriction of MSAC to recommending only 'medical' services means that interventions by other health care providers are not considered for listing.

PBAC and MSAC focus on particular drugs or 'medical services' (procedures or diagnostic tests), respectively. Generic consultative medical services (time based) have not been assessed, and listing is by medical specialty (psychiatry, emergency, and other). Some 'super-specialty' services are assessed and funded on the basis of management of a 'condition', but the need for patients to travel to these national centres means that the time period of such management is limited to a window surrounding an acute intervention.

Evaluation of effectiveness and cost-effectiveness of preventive services is undertaken, if at all, in the context of programme funding or *ad hoc* university-based research projects. The cost-effectiveness of vaccines is the only exception, and these now are considered by the PBAC.

Use of costing thresholds

None of the processes described uses an explicit threshold for cost-effectiveness, although most work on 'rules of thumb'. George et al. (2001) have analysed decisions by the PBAC between 1992 and 1996 to infer its thresholds, concluding that while there was no explicit threshold PBAC was unlikely to recommend a drug if the additional cost per life-year gained exceeded A$76,000, and was unlikely to reject a drug if the additional cost per life-year gained was less than A$42,000 [18].

Where a proposed listing of a drug is expected to add A$10 million or more to the cost of the Pharmaceutical Benefits Scheme, it also undergoes a whole-of-government consideration.

Evidence base for CEA

Clinical evidence is derived from systematic reviews for nearly all of the HTA/listing organisations/processes described. Economic evidence, however, is generally modelled using information on Australian practice patterns, prices, *etc.* None of the processes described above has the capacity to commission new/primary clinical research, and there is little co-ordination with existing medical research priority-setting processes.

Evidence is characterised using the Australian National Health and Medical Research Councils (NHMRC) recommendations, which designate six levels of evidence reflecting the effectiveness of a given design to answer a particular research question. Effectiveness is based "on the probability that the design of the study has reduced or eliminated the impact of bias on the results" [19]. The six levels of evidence for intervention studies are thus given as:[6]

Table 2: Levels of evidence

Level	Intervention
I	A systematic review of level II studies
II	A randomised controlled trial
III-1	A pseudo-randomised controlled trial (i.e. alternate allocation or some other method)
III-2	A comparative study with concurrent controls (non-randomised experimental trial; cohort study; case-control study; interrupted time-series with a control group)
III-3	A comparative study without concurrent controls (historical cohort study; two or more single-arm study; interrupted time-series without a parallel control group)
IV	Case series with either post-test or pre-test/post-test outcomes

PBAC rarely recommends drugs on less than RCT evidence, since RCT generally is regarded as the 'gold standard' for research evidence, while MSAC and the State mechanisms consider 'best available' evidence in certain circumstances.

Data base for CEA

The PBAC requires applicants to prepare detailed submissions providing evidence of effectiveness and cost-effectiveness, and these are then subject to rigorous assessment by HTA organisations contracted to PBAC and provided as confidential reports. Documentation for PBAC recommendations are considered to be 'commercial in confidence'.

[6] A detailed description of the NHMRC recommendations is provided at http://nhmrc.gov.au/publications/_files/levels_grades05.pdf.

Most submissions to PBAC use cost-minimisation or cost-effectiveness (including cost-utility) analyses. The main comparator usually is the existing therapy (e.g. a PBS-listed drug) that the new therapy proposes to replace. Patient health outcomes are usually measured in terms of mortality rates, or intermediate (surrogate) indicators such as life-years saved or quality adjusted life years (QALYs). Cost-per-QALY provides reassurance for decision-makers that there is some internal consistency in the set of decisions they have taken. This may, however, be false reassurance, as there are many different ways to deriving QALY values, and there are some inconsistencies in reported values for some health states. Thus, MSAC has had at least one lively discussion about the plausibility of reported QALY values for loss of sexual potency in interventions for benign prostatic hypertrophy and cancer of the prostate (Box 1). 'Best available' data also leads to intermediate clinical outcomes being considered in cost-effectiveness analyses.

Box 1 CEA of brachytherapy for the treatment of prostate cancer

In 2000 MSAC conducted a review of the safety, effectiveness and cost-effectiveness of brachytherapy for prostate cancer, comparing brachytherapy with three alternative treatments (radical prostatectomy (RP) and external beam radiation therapy (EBRT) and deferred treatment ('active surveillance')). The report noted the profile of adverse events differed between the treatments, with "brachytherapy possibly resulting in a higher rate of potency preservation than the comparators". A basic costing analysis estimated an additional direct cost of $3500 per patient for brachytherapy and based on these findings MSAC recommended interim public funding of brachytherapy for patients with early localised prostate cancer. The 2000 assessment was updated in 2005 to take account of evidence that had been published since.

The updated report provided estimates of direct costs of brachytherpy treatment relative to RP and ERBT (for an Australian setting), with the expected costs of brachytherapy exceeding those of RP or ERBT by between $3900 and $4750. In line with a 2003 review by NICE (UK), the review did not identify evidence for treatment effects on survival, but indicates differences in adverse events rates. Building on the NICE review that undertook modelling of expected QALYs that allowed for differences in adverse event rates in 65-year-old patients with moderately differentiated tumours, and that estimated highers expected QALYs for brachytherapy than the alternative treatments, the MSCA review noted that "[t]his gain in QALYs is, however, highly sensitive to modelled adverse event rates, with brachytherapy less effective than each of the other strategies in a worst case scenario." Based on this and other evidence, MSCA thus concluded that further research into comparative treatment effects on adverse events as well as survival was needed "before conclusive recommendations can be made about the effects, costs or costeffectiveness of strategies." The Committee thus recommended that interim public funding be continued for patients with prostate cancer that meet specific criteria.

Source: Medical Services Advisory Committee. Brachytherapy for the treatment of prostate cancer. MSAC application 1089. Assessment report. Canberra: MSCA, 2005 (available at www.msac.gov.au/internet/msac/publishing.nsf/Content/1089-1/$FILE/app1089.pdf).

MSAC requires less rigorous submissions than does PBAC, as it undertakes its own assessment, also using contracted HTA organisations, to prepare reports that form the basis of any recommendation to the Minister. These reports are published once the Minister has made a decision about listing.

Use of marginal cost-effectiveness

Where there is reliable clinical evidence of differences in effectiveness amongst patient sub-groups, this forms the basis for recommendations on the 'indications' for subsidy of drugs and medical services. Where such evidence is lacking, the two national committees (PBAC and MSAC) have been reluctant to go beyond the clinical evidence in estimating cost-effectiveness.

Use of ethical weights

PBAC has a formal policy on the 'Rule of Rescue' but ethical considerations of the other HTA processes described here are undertaken *ad hoc*, and are undocumented. The 'Rule of rescue' applies in exceptional circumstances in favour of including a drug into the benefits package. 'Exceptional circumstances' are defined as (1) no existing alternative treatment, (2) a severe and progressive medical condition, expected to lead to premature death, and (3) the medical condition applies to a small number of people. It is understood that the 'rule of rescue' "supplements rather than substitutes for the evidence-based consideration of comparative cost-effectiveness". [7]

Additional notes:

Spending on all pharmaceuticals in 2003-04 accounted for 14.4% of the total health expenditure and has risen steadily through the 1990s and the early 2000s [17: 294].

The rapid growth of medical technology in diagnostics, procedures, prostheses, devices and pharmaceuticals, and the consequent rise in expenditure on medical technology, prompted the Australian Government to commission a research report from the Productivity Commission 'Impact of Advances in Medical Technology in Australia' (2005).

The process of health technology assessment (HTA) is well-established in Australia, which is said to be a world leader in using HTA to inform funding decisions and in introducing economic assessments as part of the HTA process, which may also involve horizon scanning, technology assessment, and monitoring and review [14: 211]. Formal HTA processes for pharmaceuticals date back more than 40 years but were not introduced until the early 1980s for other medical technologies, such as procedures and devices.

[7] See http://www.aodgp.gov.au/internet/wcms/publishing.nsf/content/pbacguidelines-index~pbacguidelines-part2~pbacguidelines-part2_f.

Italy

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Formal status of the programme and/or organisation and/or institute performing CEA of existing services

Several government and arm's-length bodies are currently experimenting with the use of cost-effectiveness analysis (CEA) of pharmaceuticals and medical devices as a means of defining the formal benefits package of services provided by the Italian National Health Service (NHS). Since 2004, the central government, through the National Commission for the Definition and Updating of the Minimum Level of Benefits Package (*Commissione nazionale per la definizione e l'aggiornamento dei Livelli essenziali di assistenza e delle prestazioni in essi contenute*), has been defining a minimum package of services which have to be provided by the NHS in each of Italy's 20 regions. The Commission, also referred to as the National LEA Commission (*Livelli Essenziali di Assistenza* or 'essential levels of care'), comprises six experts of health care management, planning and organisational sciences nominated by the Ministry of Health, seven regional representatives and one representative from the Ministry of Economy and Finance [20].

In February 2007, the Italian government launched the National System for the Review and Control of the Health System (*Sistema Nazionale di Verifica e Controllo sull'Assistenza Sanitaria,* SIVeAS). The programme is mandated to assess the cost effectiveness of interventions and services included in the official benefits package. The scope of the programme includes reviewing existing services over an initial phase of three years and developing guidelines and standards for using CEA in the context of making decisions about publicly-funded health services.

Other key organisations involved in CEA (for various purposes) include:

- The Commission for Medical Devices (*Commissione Unica dei Dispositivi medici*), a technical arm's-length body which undertakes cost benefit analysis of existing and new medical technologies.

- The Italian Drug Agency (*Agenzia Italiana del Farmaco*), in particular its Technical Scientific Commission, which increasingly applies CEA in order to classify drugs for public reimbursement.

- The Agency for Regional Health Care Services (*Agenzia dei Servizi Sanitari Regionali, ASSR*) co-funds a research project on CEA which aims to develop guidelines for screening. This project also involves several universities, health care organisations and regional agencies.

- Regional Agencies for Health and Heath Care Organisations may propose research programmes that involve CEA of specific interventions to the Minister of Health and to the Agency for Regional Health Care Services (ASSR).

- Some regions (Emilia-Romagna, Marche, Toscana and Piemonte) have established regional organisations which undertake CEA for regional planning and policy making.

In addition to government organisations there are several independent research institutes, associations, health care organisations, universities and private firms involved in assessing the utilisation of drugs, health technologies and procedures also using CEA.

Remit of the programme

The overall remit of (official) organisations involved in CEA includes health technology assessment, horizon scanning of innovative technologies, medical devices, drugs and specific procedures (i.e. therapies or treatments). The main task of SIVeAS will be to review and assess services included in the mandatory benefits package.

Funding sources

Reflecting the devolution of responsibilities of the centre to the regions, the funding of activities has been devolved, with some organisations funded by the Ministry of Health and others by the Agency for Regional Health Care Services. Some activities are co-financed by pharmaceutical companies or health care enterprises together with health care organisations, universities and, in some cases, non-profit organisations.

Use of recommendations to determine the nature and scope of the benefits package

The National LEA Commission, the Italian Drug Agency and the Commission for Medical Devices of the Regions provide recommendations on the nature and scope of the formal benefits package, including guidelines for health service providers. However, only the of by the National Commission and the Italian Drug Agency are mandatory for the regions.

Agencies responsible for decision-making about the nature and scope of the benefits package

Several organisations, representing national and regional authorities, are involved in decision-making. At the national level, these include the Ministry of Health, specifically its Department of Planning and Quality of Health Care Services, and the National LEA Commission. Regional decision making is integrated through the State-Regions agreement (*Conferenza Stato-Regioni*).

Scale of the programme's ambition

The scale of the new programme (SIVeAS) has not yet been defined. The Ministry of Health will take the lead in setting the agenda for the new programme in February 2007.

Volume of financial resources dedicated to the programme

SIVeAS has been allocated €10 million per annum for the first three years.

It is not possible to quantify the volume of financial resources used to assess CEA of interventions across all relevant organisations. The largest funder of CEA activities is the government, with regional agencies and other organisations providing additional funding.

Human resource capacity dedicated to the programme

The human resource requirements of SIVeAS have yet to be specified. It is anticipated that most professionals working in this programme will have a background in medicine and pharmacoeconomics.

Selection process of interventions for CEA

Criteria for selection of interventions to undergo CEA under the SIVeAS programme have not yet been defined. A rapid assessment of previous analyses suggests that interventions to be assessed through CEA are selected based on the incidence of the related condition, the costs involved in the provision of drugs and preventive procedures and the novelty of the procedure.

At present, assessments of interventions using CEA tend to focus on procedures or drugs.

Use of costing thresholds

At present, there are no costing thresholds that determine whether a selected intervention is to be excluded from the publicly-financed benefits package. However, potential approaches are currently being explored.

Evidence base for CEA

So far, analyses using CEA have been limited to systematic reviews. Primary data collection in the form of research projects is rare in Italy. It is expected that new research will be commissioned involving CEA of existing services; the findings of this research are expected to be published within the next three years.

Evidence used for CEA comes mostly from international sources, mainly based on randomised controlled trials (RCTs). Few data are from RCTs conducted in Italy.

Data base for CEA

A recent review of pharmacoeconomic studies in Italy suggests that only 8% of the data used were derived from primary research [21]. Most studies used direct cost data, as well as epidemiological and administrative data. Studies typically assessed cost per Quality Adjusted Life Years (QALY) or per Disability Adjusted Life Years (DALY) or per Life Year Gained (LYG). The authors of the review highlighted the challenges of using evidence from pharmacoeconomic studies to inform decision-making due to the heterogeneity of methods and data used, along with sponsor interference with results. The review highlighted the need for guidelines and better-defined standards if CEA is to be used to inform the definition of the public benefits package.

Use of marginal cost-effectiveness

There have been attempts to assess the marginal benefit for different patients groups, but the scope of available data is limited.

Use of ethical weights

There is no indication that ethical weights have been considered so far.

New Zealand[8]

Formal status of organisations or programmes performing CEA of existing interventions

The Pharmaceutical Management Agency (PHARMAC) is a stand-alone Crown entity and is directly accountable to the Minister of Health. Set up in 1993 PHARMAC under the 1993 Health and Disabilities Services Act to improve the management of Government expenditure on pharmaceuticals it was initially owned by the Health Funding Authority and the Regional Health Authorities before it received its current status under the New Zealand Public Health and Disability Act 2000.

Remit of the organisation

PHARMAC's mandate is "to secure for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the funding provided" as outlined in the 2000 Public Health and Disability Act [23].

A key function is to maintain and manage the list of pharmaceuticals subsidised by the Government, the *Pharmaceutical Schedule*. In 2001, it was also authorised to manage the purchasing of hospital pharmaceuticals on behalf of District Health Board. It manages the *Exceptional Circumstances* (EC) scheme, which allows for medicines not normally subsidised to be funded for rare and unusual conditions. In addition, PHARMAC is mandated to promote the responsible use of pharmaceuticals and to develop guidelines on pharmaceutical prescribing.

Funding sources

PHARMAC's operations are funded from the health budget (i.e. from taxes). The amount of funding for its operations is negotiated annually with District Health Boards.

Use of recommendations to determine the nature and scope of the benefits package

Decisions on the inclusion or exclusion of medicines in/from the Pharmaceutical Schedule are made by the PHARMAC Board [[23]]. The Board comprises up to six members who are appointed by the Minister of Health. All decisions relating to PHARMAC's operation are made by or under the authority of the Board.

Board decisions are informed by independent medical experts represented at the Pharmacology and Therapeutics Advisory Committee (PTAC), its specialist sub-

[8] Information presented in this case study is based, primarily, on [22]. The report updates an earlier report, published in 1997; it is available as draft only and has been under consultation until October 2006. The questionnaire was reviewed and amended by Rachel Grocott, Senior Analyst, Hospital Pharmaceuticals Assessment, Pharmac.

committees and PHARMAC staff. Views of patients and consumers are considered in the decision-making process through the Consumer Advisory Committee (CAC).

PHARMAC's decisions are based on nine explicit criteria [23]:.

- "the health needs of all eligible people within New Zealand (eligible as defined by the Government's current rules of eligibility)
- the particular needs of Maori and Pacific peoples
- the availability and suitability of existing medicines, therapeutic medical devices and related products and related things
- the clinical benefits and risks of pharmaceuticals
- the cost-effectiveness of meeting health needs by funding pharmaceuticals, rather than by using other publicly funded health and disability support services
- the budgetary impact (in terms of the pharmaceutical budget and the Government's overall health budget) of any changes to the Pharmaceutical Schedule
- the direct cost to health service users
- the Government's priorities for health funding, as set out in any objectives notified by the Crown to PHARMAC, or in PHARMAC's Funding Agreement, or elsewhere, and
- any other criteria that PHARMAC thinks are relevant. PHARMAC will carry out the necessary consultation whenever it intends to take any 'other criteria' into account."

Organisations responsible for decision-making on the nature and scope of the benefits package

Decisions about inclusion/exclusion of pharmaceuticals on the Pharmaceutical Schedule are directly made by the PHARMAC Board. PHARMAC decisions are binding for DHBs and do not require ministerial approval.

Scale of organisation's ambition

Pharmaceutical suppliers may apply to PHARMAC to have a pharmaceutical product listed on the Pharmaceutical Schedule for public subsidy. A key instrument for evaluation is cost-utility analysis (CUA) which is used as a means to assess the relative cost-effectiveness of a pharmaceutical compared to other funding options. The analysis is undertaken from the perspective of the funder (i.e. for health services provided in the public sector) [22]. The majority of new applications require a cost-utility analysis (CUA), which is done 'in-house' by PHARMAC staff.

On occasion, pharmaceutical suppliers undertake CUAs in consultation with PHARMAC staff. Between 2000 and July 2006, PHARMAC completed about 120 funding analyses (approximately 20 per annum), of which 35% were rapid, 21% preliminary, 28% indicative and 13% detailed (see below) [22].

Number and level of detail of CUAs undertaken by PHARMAC varies from depending on the available budget for funding pharmaceuticals (PHARMAC operates under a fixed pharmaceutical budget), the number of new applications (and level of financial risk involved), and staff resources.

Volume of financial resources dedicated to the organisation

In 2006, PHARMAC's total expenditure for all of PHARMAC's operations was reported at NZD 13,234,000 (roughly GBP 4,000,000), which was within the calculated budget of NZD 15,571,000 (GBP 5,200,000). Expenditure included operating costs, salaries and related costs, audit fees, directors fees, depreciation, rentals and leases, and programmes related to high cost medicines and responsible use of pharmaceuticals.[24]

The estimated cost to PHARMAC of undertaking cost-utility analysis is approximately two full-time equivalents (approximately NZ$300,000 including overheads) (Grocott, Pharmac, personal communication).

Human resource capacity dedicated to the programme

As noted above, most assessments of pharmaceuticals are undertaken in-house at PHARMAC by a small team of usually three analysts who have a range of backgrounds, including economics, pharmacology and public health medicine. These analysts are also involved in other areas of work at PHARMAC.

Selection process of interventions for CEA

Applications for new pharmaceuticals to be listed on the Pharmaceutical Schedule or for expanding access to pharmaceuticals are usually initiated by pharmaceutical suppliers, although they can also be initiated by members of public. Following receipt of a funding proposal, the clinical evidence is reviewed by an independent group of medical practitioners, the Pharmacology and Therapeutic Advisory Committee (PTAC). The committee makes recommendations to the PHARMAC Board for the assignment of high, medium or low priority of proposals for further evaluation, or that a proposal be declined, referred back to the supplier for further information, or referred to a subcommittee to clarify whether the application meets the criteria for evaluation . Based on the PTAC prioritisation (and any other relevant criteria such as level of analysis required and stage of the negotiation process with the pharmaceutical supplier), pharmaceuticals are then prioritised in house for cost-utility analysis.

PHARMAC only evaluates pharmaceuticals (i.e. not other health technologies). However, the comparator used in cost-effectiveness analyses is current clinical practice, the evaluation might include medical procedures also [22].

Use of costing thresholds

There is no threshold below which a pharmaceutical is considered 'cost-effective' and thus included in or above which it is automatically excluded from the Pharmaceutical Schedule [22]. Cost-effectiveness is only one of the nine criteria PHARMAC applies for decision-making. In addition, whether or not a product is considered cost-effective will also depend on the range of additional pharmaceuticals that could be funded within the current budget and the amount of funding available. This will inevitably vary over time.

Normally, orphan drugs and high cost medicines are treated no differently to other pharmaceuticals considered by PHARMAC. The higher cost of selected medicines should not form justification in itself to adopt a different funding approach (see also below) [25].

Evidence base for CEA

The evaluation of pharmaceutical by PHARMAC uses, and critically appraises all available clinical evidence. Priority is given to the best available evidence, i.e. well-conducted randomised controlled trials (RCTs) and meta-analyses for the use in CUA when estimating relative treatment effects. In the absence of valid RCTs, evidence from the best available study designs is considered next. PHARMAC staff undertake systematic reviews of the literature and, if necessary, also meta-analyses.

PHARMAC primarily undertakes cost-utility assessments (CUA), a subcategory of cost-effectiveness analysis (CEA). CUAs are predominantly based on systematic reviews of evidence. PHARMAC provides analyses at different level of detail [22]:

- detailed (within 3-6 month of two full-time staff equivalents on average),
- indicative (within 4-6 weeks),
- preliminary (within 1-2 weeks), and
- rapid (within 1-2 days).

The level of analysis is determined by a range of factors, including the urgency of the evaluation, the expected impact on pharmaceutical budget, the reliability of results, the extent of information available for analysis, the impact of CUA on the actual funding decision, and the availability of staff time. Only few proposals receive detailed evaluation. Although possible, primary research is rarely commissioned.

Data base for CEA

The conceptual base of CUA is cost per Quality-adjusted Life Year (QALY). It is recommended that utility values be derived from the EQ-5D New Zealand Tariff 2, a generic tool based on a 1999 New Zealand population survey.[9] Subjective judgements on the value of health states are to be validated either through published literature or expert clinical input. If patient adherence is considered to be relevant to the effectiveness of a treatment adherence rates are also taken into account.

The comparator used in CUAs is usually current clinical practice, which may or may not be the most effective treatment. This is compatible with PHARMAC's principle that the assessment is undertaken from the perspective of the funder.

Clinical data are used to estimate the relative treatment effect, the baseline risk of disease/ natural history/ prognosis, and in some cases, health-related quality of life.

[9] The New Zealand EQ-5D differentiates two tariffs: Tariff 1 includes 'logical inconsistencies' inherent in the dataset of the survey (i.e. missing and implausible responses); these were excluded in tariff 2.

Cost data include costs associated with pharmaceuticals, inpatient hospital services, outpatient hospital services and direct patient costs. Indirect patient costs, such as the cost of premature mortality or of time off work; direct non-health care costs (e.g. costs to other public sectors, direct and indirect taxes and transfer payments) and indirect health care costs (e.g. future health care costs, capital costs, depreciation and overhead costs) are not included in CUAs undertaken by PHARMAC. Cost data considered in a CUA should meet the following requirements [22]:

- Pharmaceutical costs should take into account the price and dose of a pharmaceutical, dispensing fees and pharmacy mark-up, cost of administering a pharmaceutical and costs of co-administering with a treatment where this is required. *Sources*: Pharmaceutical Schedule; pharmaceutical suppliers (patent information); clinical trials (doses); DHB hospitals.

- Hospital inpatient costs are calculated using diagnosis-related group (DRGs) prices and classification. Although DRG prices provide an approximation to real costs only information on average cost is "more readily available and in most cases sufficient" [22]. Adjustments are made for highly complex conditions, volume of patients, services provided in tertiary care and in rural areas, and mechanical ventilation. Hospital costs and savings are disaggregated into several categories, including real cost savings, nominal cost savings and additional costs (e.g. for additional tests or procedures involved in administering a new drug*). Sources*: New Zealand Health Information Service (NZHIS).

- Hospital outpatient costs include outpatient clinic services, laboratory and diagnostic tests, nursing services provided by the hospital, and costs of hospital-based outpatient programmes. Outpatient costs also include 'terminal costs', i.e. costs incurred during the final phase of a patient's life, in cases where patients receive palliative care until death outside hospital. *Sources*: DHB hospitals; NZHIS.

- Direct patient health costs are restricted to health care costs which are only partly subsidised by the government, i.e. require co-payment such as for visits to a general practitioner, for pharmaceuticals and for home or continuing care. *Sources*: Ministry of Health.

Use of marginal cost-effectiveness

The target population is defined as the New Zealand population most likely to receive treatment. Subgroup analysis is used if specific subgroups are likely to specifically benefit from a treatment and if these groups can be successfully identified and targeted through funding mechanisms. Subgroups should be defined a *priori* in the clinical trial.

Use of ethical weights

PHARMAC does not include 'ethical weights' in analyses to give priority to certain treatments or conditions. PHARMAC considers that it is important for the results of CUAs to be as value-free as possible. Also, as noted earlier, cost-effectiveness forms only one of nine decision-making criteria of PHARMAC [22]

As a relevant aside, PHARMAC is currently consulting on its approach to the funding of high cost medicines, namely how and on what basis should PHARMAC make decisions on funding "high cost" medicines, and in particular whether high cost medicines require a different approach to their funding [25]. As outlined earlier, PHARMAC's preliminary conclusion to date is that there are no persuasive arguments for treating the funding of 'high cost medicines' differently to other medicines. The ongoing consultation seeks public views on how PHARMAC should approach the trade-off between funding the treatment of very small numbers of patients requiring very expensive medicines (for very rare conditions) against the treatment of large numbers of patients requiring less expensive medicines (for more common conditions), to be interpreted in light of independent input obtained from a range of experts (consultation to be closed by 5 March 2007).

Sweden

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Formal status of organisations and programmes performing CEA of existing interventions

CEA of new and existing interventions are primarily undertaken by three national authorities: The Swedish Council on Technology Assessment in Health Care (SBU), the National Board of Health and Welfare (NBHW) and the Pharmaceutical Benefits Board (LFN).

To some extent CEA is also used at the local level, e.g. by some formulary committees mandated by county councils. However, these approaches are not systematically documented and do not constitute national programmes.

The Pharmaceutical Benefits Board (LFN), established in October 2002, determines whether prescription drugs used in outpatient settings should be publicly-funded. LFN is also responsible for negotiating prices with pharmaceutical suppliers and setting nationally uniform prices.[10] Funding decisions made by LFN are primarily based on CEA [5]. In addition to the assessment of new pharmaceuticals, LFN also reviews drugs that were approved before it was established.

The National Board of Health and Welfare (NBHW**)** develops guidelines for priority setting and clinical decision making in major therapeutic areas. The cost effectiveness of interventions is systematically assessed and taken into account.

The Swedish Council on Technology Assessment in Health Care (SBU) undertakes health technology assessments and systematic reviews which include studies on cost effectiveness. CEA plays a minor role in the work of SBU.

Remit of organisations and programmes

The remits of the organisations are outlined above.

Decisions about public reimbursement of services are based on three principles of priority setting in health care, as determined by the Swedish Parliament in 1997 (Swedish Health Care Act, para 2):

- The *principle of human dignity*: health care shall be provided in a spirit of respect for the equal value of all human beings.
- The *principle of need and solidarity*: those with the greatest medical need take precedence over those with less severe conditions as it relates to health care resources, for example the reimbursement of drugs.

[10] Pharmaceuticals used by hospitals solely for inpatient care are exempt from national pricing as hospitals can negotiate prices directly with the supplier [5].

- The *cost-effectiveness principle*: the costs for using a medicine should be reasonable from a medical, humanitarian, and socioeconomic perspective.

Funding sources

All the organisations listed above are national agencies and funded through taxes.

Use of recommendations to determine the nature and scope of the benefits package

Decisions by LFN to list or de-list a drug (i.e. to define its eligibility for reimbursement) are binding. Consequently the benefits package and prices of prescription drugs are uniform across the country.

Guidelines developed by NBHW are not binding; rather, they constitute a tool to support listing/de-listing decisions at the local level (i.e. by county councils).

SBU provides reviews and information but is not involved in issuing guidelines or directives.

Organisations responsible for decision making about the nature and scope of the benefits package

Decisions at the national level are restricted to prescription drugs (outside hospital) only, as made by LFN. All other decisions regarding the listing, reimbursement and pricing of interventions and services are the responsibility of the county councils.

The 21 county councils play a dominant role in Swedish health care. According to the Health Care Act of 1982, “every county council has to offer good health and medical services to persons living within its boundaries” and “promote the health of all residents”. However, county councils are not responsible for nursing home care for older people; this function has been devolved to the 290 municipalities which are equally autonomous in determining the delivery of services.

County councils and municipalities levy proportional income taxes on their respective resident populations; these taxes constitute 70% of their total revenue. Funding of health services is complemented by central government grants and user charges. Government grants are based, in part, on an allocation formula that redistributes resources between municipalities and county councils based on demographic, geographic and socio-economic indicators. By this means the central government aims to minimise regional variation in health care standards and access. Prescription drugs and dental care are funded through national social insurance (largely funded through employer contributions [5]). However, since 1998 the central government has been allocating budgets for pharmaceuticals to the county councils to allow local control of pharmaceutical expenses.

Scale of the organisations’ ambition

NBHW reviews interventions by therapeutic area such as cancer (which is being reviewed at present). These reviews are expected to take several years per therapeutic

area (2-3 years for cancer). As new interventions are introduced, previous assessments will require updating.

LFN assesses new pharmaceuticals upon application by pharmaceutical companies. As noted above, LFN also reviews drugs that were approved for public reimbursement prior to October 2002. This review includes some 2000 drugs and assessments are undertaken by therapeutic group (see document 1). A total of 49 groups of pharmaceuticals have been identified and prioritised for review. Since 2003, LFN has completed a review of the reimbursement status of two groups (medicines used for the treatment of migraine and antacids; summary documents of these reviews are enclosed as documents 2 and 3). Forthcoming treatment areas subject to review are: high blood pressure, asthma, depression and high cholesterol. While there is no precise timetable for the review process it is expected that this work will continue for a number of years.

Volume of financial resources dedicated to the organisations

SBU receives an annual budget of SEK 50 million (about £ 3.6 million). There is no information available on the budgets of the other two agencies.

Human resource capacity dedicated to the organisations

NBHW normally contracts clinical experts and/or health economists who are experts in the topic area to be reviewed. Reviews also usually involve a larger number of external experts; this is seen as a strategy for improving the implementation of findings.

LFN employs a professional team of medical experts and health economists; reimbursement decisions are taken by LFN's board which is comprised of a chairperson and ten members appointed by the government for a period of two years [26]. The board includes two representatives of patient and consumer groups [27].

For reviews of new drugs, pharmaceutical companies submit an application for their product to be considered for reimbursement. As part of the application companies are required to provide economic evaluations and detailed documentation on the effectiveness and cost-effectiveness of the drug (LFN guidelines enclosed as document 4). Reviews of the reimbursement status of medicines often require additional modelling and analysis of data, which is normally undertaken by LFN personnel.

Selection process of interventions for CEA

Prioritisation of areas and services subject to review by LFN (i.e. reimbursement status of existing drugs) or for guideline development through NBHW is by major disease or therapeutic group (these are usually related). According to LFN the selection of migraine medicines and antacids as the first two pharmaceutical groups to be reviewed for reimbursement status was to "test working methods and organisation" and other aspects of the review process [26]. The selection of groups considered for the next review stage is more explicitly determined by the sales value of respective medicines, i.e. those that contributed most to the pharmaceutical budget (reference year: 2003) will be reviewed first (see above).

Assessments of new pharmaceuticals to be considered for reimbursement are carried out as requested, following an application by a pharmaceutical company.

In general, the pharmaceutical benefit scheme is product-orientated. Consequently, it is the cost effectiveness of products rather than indications that is to be assessed. LFN may, however, decide to limit reimbursement of a drug to certain indications and/or sub-groups of patients, even if the drug has previously been licensed for a broader indication or patient group.

Use of costing thresholds

Existing CEAs do not use explicit costing thresholds. However, empirical studies (such as on road safety interventions and some smaller studies on health care) have shown that there is an implicit threshold of SEK 500,000 per QALY (about £36,000) (Anell, unpublished) This threshold is not absolute as other criteria, for example need for treatment, are also taken into consideration. Thus, several orphan drugs have been approved for public reimbursement by LFN despite a limited evidence base regarding cost-effectiveness and the fact that the related costs of treatment may be high on a per patient/QALY basis. At the same time it was recognised that only a small number of patients normally receives the relevant treatment and it was therefore assumed that the overall impact on the pharmaceutical budget would be modest [27].

Evidence base for CEA

The development of guidelines by NBHW is largely informed by systematic reviews, for example those undertaken by SBU. If the published evidence is considered as insufficient for guideline development, NBHW will apply its own assumptions and analyses.

Evaluations of new drugs undertaken by LFN are normally based on the documentation provided by the pharmaceutical company (see LFN guidelines in the Annex). Reviews of the reimbursement status of existing drugs usually use existing documentation, complemented by additional modelling and analyses.

All reviews are generally based on the best available evidence, with RCTs being considered as the ‘gold standard’.

Data base for CEA

The attached LFN guidelines for economic evaluation of new and existing medicines give an overview of the nature and scope of data and the conceptual base used. Data and underlying concepts apply equally to new and existing drugs. NBHW uses the same standards.

Use of marginal cost-effectiveness

Marginal cost-effectiveness for different patient groups is considered important. Indications for reimbursement are frequently limited to those patients groups that are likely to benefit most and have the highest risk of complications. This applies in particular to interventions that affect large population groups, such as the treatment of diabetes or high cholesterol [27].

Use of ethical weights

'Ethical' considerations are not formally included as part of the economic analysis. However decisions about the reimbursement status of drugs are not solely based on economic considerations; other criteria such as clinical need and the 'degree of priority' are also taken into account [27]. For example, reimbursement of the treatment of erectile dysfunction was rejected on grounds of 'low priority' even though the treatment may be regarded as cost-effective for some patient groups. However, this decision was controversial within the LFN board and three members voiced concerns against the decision. The process of decision-making at LFN is not entirely transparent.

Part II

Netherlands: Reviewing health care benefits

The Netherlands has a long tradition of using information on cost-effectiveness to inform decisions on the benefits package of health services funded through the social health insurance system. Using the broader label of technology assessment, CEA was introduced in the early 1980s by the Health Care Insurance Board (CVZ, *College voor Zorgverzekeringen*, previously: *Ziekenfondsraad*) [28]. A dedicated agency, the Fund for Investigative Medicine (*Fonds ontwikkelingsgeneeskunde*), administered by CVZ was set up in 1988. The Fund essentially became the main Dutch HTA programme that, with an annual budget of approximately € 16 million (from the Ministries of Health, Welfare and Sports, and of Education, Culture and Science), was mandated with funding projects that evaluate new or established technologies. The Fund has recently been incorporate in the Netherlands Organisation for Health Research and Development (ZonMW), which has been funding HTA research previously. There are several other initiatives both at the national and local level undertaking HTA, for example the Health Council of the Netherlands (Gezondheidsraad) issues HTA reports on a regular basis [29].

The majority of evaluations focus on new pharmaceuticals and procedures; however, there have been recurring attempts to revisit the health care benefits package with the view to potentially exclude selected services from the public system so as to ensure essential services for all. Thus, in 1991, the Dutch Committee on Choices in Health care published the so-called Dunning Report 'Choices in health care' (*Kiezen en Delen*), which described a framework aimed at assisting policy makers to determine the basic health care benefits provided under social health insurance [30]. Building on a set of core principles arguing, for example, that explicit and publicly accountable choices are preferable over 'covert rationing', it formulated four criteria or "filters" on which decisions on a basic benefits package should be based:

1. Is the service necessary (from a community point of view)?
2. Has the service/intervention been demonstrated to be effective?
3. Has the service/intervention been demonstrated to be efficient?
4. Can the payment for the service be left to the responsibility of the individual? [28]

Together, these criteria were referred to as "Dunning's Funnel", because of their hierarchical nature. Thus, priority setting should first involve determining if a service was necessary. If the answer was yes, then decision-makers should proceed to asking whether or not it was effective etc. The criteria elicited considerable debate, in particular criteria 1 and 4 and the implied intention to exclude services from funding at the macro level. Thus, Ham (1997) observed that "[i]t was this [debate] that forced the Dutch Government to withdraw proposals to exclude contraceptives from the insurance package" and which eventually resulted in decisions on the benefits package abstaining from excluding entire groups of services but limiting the extent to which selected

services would be reimbursed under the public system through for example defining age thresholds for entitlement (e.g. IVF for women under the age of 40 only) [30].

While the Dunning report has as yet to be implemented fully in practice it did trigger several developments that appear to have strengthened a more systematic approach to priority setting for technologies and interventions to undergo economic evaluation [28]. Of particular relevance in this context appears to be a Delphi-exercise initiated by the Health Care Insurance Board in 1993. Involved some 30 experts in the Netherlands, this exercise generated a list of 126 routinely used services or technologies that were considered as of doubtful cost-effectiveness. These technologies were then ranked according to a set of criteria:

- degree of uncertainty as it relates to efficacy, effectiveness or efficiency;
- frequency of use;
- costs;
- impact on health outcomes (potential to decrease morbidity or mortality or to increase quality of life);
- potential impact of technology assessment on rate of use of that technology.

The top five priorities thus identified for further assessment were: ultrasound treatment of problems of the locomotive system; treatment and cure of non-hospitalised psychiatric patients; specialist care for chronic conditions; diagnosis of suspected hernia nucleus pulposa; and diagnostic arthroscopy of the knee compared with diagnostic MRI [28].

Reviewing the experience so far, Berg et al. (2004) observed that other bodies subsequently produced other rankings, and noted that while this has helped to shape the agenda for HTA funding and research the overall 'system' of the application and use of HTA in the Netherlands may be considered as fairly loose. While HTA, and more specifically, CEA is being undertaken quite widely, and has become an important component of academic medical research, its influence in informing decision making as it relates to health care funding has remained rather patchy. Thus, Berg et al. (2004) noted that "the link between the results of [HTA] studies and the health-care choices implemented by government (or insurance companies, for that matter) remain […] partial." More specifically, they noted that, in the Netherlands "HTA analyses are sometimes explicitly performed to guide national policy, and are increasingly drawn upon to back up (or influence) appraisal processes. Yet, the list of excluded services is still minimal and highly eclectic." [28]

This situation might however change. Following the health care reform in 2006 the remit of the CVZ will be revised; and this may include an expansion of its mandate to reviewing existing health care interventions. The development of the new mandate involves a review of previously used approaches and, importantly, an evaluation of options for integrating the technical assessment into the multi-stakeholder decision-making process of the Dutch health system.

It is as yet unclear whether the new policy will extend the use of cost-effectiveness analysis to decisions that may potentially lead to the exclusion of previously included interventions. However the government has indicated its interest in strengthening the role of cost-effectiveness analysis, specifically in what is called the 'opportunity

approach', i.e. for every newly included intervention another intervention should be excluded from the benefits package.

New Zealand: Defining 'core services' and prioritising patients

Since the 1990s New Zealand has experimented with approaches to defining patients' access to and eligibility for public health services. This has taken place in the context of resource constraints but also as a means to address longstanding issues such as long waiting lists and substantial regional variation in access to health services.

On the supply-side, there was an explicit attempt to define a list of 'core services' to be offered in the public sector free at the point of use; however as it appeared to be not feasible to find a consensus on a clear-cut definition these efforts were not pursued any further. As a means to manage demand the government subsequently introduced a booking system for (elective) surgical procedures, prioritising patients for treatment according to (1) the severity of their condition and (2) their potential to benefit. Both approaches make an explicit attempt to ration services and to do so in a defensible and transparent way based on explicit criteria. However, the experience shows that despite these objectives both approaches have attracted substantial debate about the legitimacy of rationing and the appropriateness of the approach.

The 'core services' experiment

New Zealand's attempt to define a package of 'core health services' to which every New Zealander has access within the public system dates back to the early 1990s. In 1992 the incoming centre-right government established a National Advisory Committee on Core Health and Disability Support Services ('Core Services Committee', CSC) which was mandated to evaluate possibilities to clarify the entitlements of patients in the public health sector.

The initiative was part of the government's plan to establish a more competitive market-based health system in which purchasers would be able to compete for patient enrolment as long as they provided a set package of publicly financed services [11]. The initiative was also intended to promote equity of access to services, to provide value for money and to reduce regional variation in service availability [31]. Cost-effectiveness of services was an explicit criterion on which a funding decision should be based. Interventions that were not part of the 'core' would be excluded from public funding and would be paid for by the patient.

The process of defining 'core services', however, proved to be difficult and highly controversial. In an attempt to increase the transparency and acceptability the process involved extensive public and expert consultation, prompting, in turn, considerable public and political debate about the fairness and feasibility of defining 'core services' that would be publicly paid for. The idea of placing a monetary value on a human life was widely rejected.

As no compromise could be found, the CSC eventually rejected the idea of defining an explicit list of services to be funded under the public system [8]. Instead it recommended the development of guidelines to support purchasers' priority-setting ("the closest they would ever get to core services" [11]). The guidelines that were eventually developed were of a general nature and included principles such as that the treatment should be of benefit, provide value for money, be consistent with community values and present a fair

use of public resources [11] (these principles also inform Pharmac's decisions on drug reimbursement).

On the clinical level, guideline development aimed to support clinicians' decisions on when to discontinue or withhold care. This moved, at least partly, the argument from cost-effectiveness to clinical considerations and shifted the responsibility for decision-making from the authorities to clinicians. The guidelines were nevertheless disputed by some clinicians.

This was illustrated by two cases of patients with end stage renal failure who were denied access to dialysis [8]. The decision to withhold dialysis was made primarily on clinical grounds (both patients had significant co-morbidities), yet it did not prevent substantial public debate about the fairness and appropriateness of denial of treatment. The decision was also in line with clinical guidelines that set out criteria for treatment. Although not solely relying on an age threshold, the guidelines recommended discontinuing treatment (i.e. dialysis) for patients over 75 years of age and with severe co-morbidities (unrelated to the renal condition) [32]. While cost-effectiveness as such was not an explicit criterion resource implications were considered relevant.

In both cases, the patients' families took legal action against the decision, complaining to the Human Rights Commission and, in one case, to the High Court. In one case (Mr McKeown 76 years, suffering from coronary artery disease and prostate cancer), the decision was eventually rejected on the basis of age discrimination, and the treatment was continued (Mr McKeown died of his co-morbidities 18 month later). In the second case (Mr Williams, 63 years) the Court of Appeal confirmed the decision to discontinue treatment stating that the decision was solely based on clinical indications and resource implications were irrelevant to the case.

Despite the controversy that attends rationing and denying of services to individual patients, there is a perception that the debate has, over time, enhanced the level of acceptance among the public of the inevitability of rationing and has led to a growing awareness that resources in health care are limited [11]. Guidelines to inform clinical decisions have been further developed, most prominently by the New Zealand Guidelines Group (NZGG). Set up by the National Health Committee (the successor body of the CSC) in 1996 as a network of expert, the NZGG has developed into an independent not-for-profit organisation with its main objective being the promotion of evidence-based medicine. As such the NZGG has assumed counselling role independent from the Ministry of Health or the CSC.

The surgical booking system

In 1993, the Core Services Committee also commissioned a report on waiting lists. The report recommended the implementation of a system that prioritises patients' access to elective services on the basis of the severity of their condition and their potential to benefit [33]. The so-called 'surgical booking system' was first tested in 1994 and rolled out countrywide in 1998.

The system is designed to manage elective surgical lists by providing clinicians and general practitioners with a guideline-based tool for decision-making. The booking

system is intended to ensure that persons with similar need for treatment have to wait a similar amount of time (maximum of six months). The system represents an attempt to replace the previous practice of 'rationing by delay' with a more objective approach to decision-making. However, as a means to manage demand, the surgical booking system also is a tool for explicit rationing [34].

The system requires hospital specialists to whom patients have been referred by their GPs to score patients' need and ability to benefit from surgery according to clinical priority assessment criteria (CPAC) using a clinical multi-criteria algorithm which produces a score for each patient out of a maximum of 100-points. There are different algorithms for each of the main elective surgical procedures. Patients whose score is above a certain threshold are booked for an appointment to receive surgery within six month. The threshold can vary based on changes in resource availability and what is regarded as an acceptable threshold severity. Patients who do not qualify for surgery according to these criteria are referred back to their primary care practitioner with a plan for patient care. These patients receive six monthly reviews to determine whether their condition has changed and are re-assessed if their condition deteriorates. The process is guided by three different types of national guidelines

(i) referral guidelines for primary care practitioners,

(ii) guidelines on access criteria for first specialist assessment, and

(iii) clinical priority assessment criteria (CPAC) for specialist assessment of whether to list for surgery (points system).

Box 1: National Clinical Priority Assessment Criteria (CPAC): Aortic stenosis

1. Severity of aortic stenosis	
AV (mean) gradient (mmHg)	maximum 30 points
AV area (cm^2)	maximum 30 points
2. Symptoms	
syncope	
angina	total maximum
breathlessness	30 points
3. LV Dysfunction (or EF)	maximum 20 points
4. Associated disease	maximum 15 points
5. LVH	maximum 5 points

Source: http://www.electiveservices.govt.nz/pdfs/aortic-stenosis.pdf (document 5)

Having been in operation for about ten years, the system enjoys increasing acceptance among general practitioners and clinicians, who have also been closely involved in the development of the methodology. At the beginning, the system faced substantial teething problems, e.g. its uneven utilisation (as different clinicians interpreted the CPAC differently and used them to different degrees), the initial absence of national standards and the lack of a framework to ensure accountability. Also, some clinicians expressed concern about the validity of the scoring system and criticised the effectiveness and appropriateness of the CPAC approach, arguing that it had not been evaluated sufficiently. Thus, the implementation of the system was slower than had been expected; however this was also partly because of the massive backlog of patients waiting for surgery. Although physicians had been involved in the development of the tool (though not consistently in all local settings), it was argued that support from physicians would have been stronger if the approach to involve clinicians had been more systematic [35].

However, the system was arguably more successful in managing demand than in reducing waiting lists (which was its primary objective). Although waiting times have fallen following the introduction of the booking system (and an injection of additional funds), a recent incident of 'waiting list culling' highlights that the problem of demand exceeding resources continues to be pertinent [36]. Patients who should have been returned to their GPs for monitoring and review had been accumulating on waiting lists even though they could not be treated within six months with the capacity available. It has been argued that this incidence demonstrates the continuing reluctance of some clinicians to use the points system as an absolute cut-off for access to surgery.

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Annex

Further reading

General

*Health*Basket project (April 2004 – March 2007), coordinated by the European Health Management Association, provides information on health benefits and service costs in 9 European countries. Country overviews and synthesis reports available from http://www.ehma.org/projects/default.asp?NCID=112.

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http://www.health.gov.au/internet/wcms/publishing.nsf/Content/Pharmaceutical+Benefits+Advisory+Committee-1

Therapeutic Goods Administration (TGA)
http://www.tga.gov.au/

Medical Services Advisory Committee
http://www.msac.gov.au/

Royal Australasian College of Surgeons (ASERNIP-S)
http://www.surgeons.org/Content/NavigationMenu/Research/ASERNIPS/default.htm

Prostheses and Devices Committee
http://www.health.gov.au/internet/wcms/publishing.nsf/Content/health-privatehealth-providers-prosths.htm

National Health and Medical Research Council
http://www.nhmrc.gov.au/

HealthPACT
http://www.health.gov.au/internet/horizon/publishing.nsf/Content/healthpact-2

Italy

Ministry of Health
www.ministerosalute.it

Italian Drug Agency
www.agenziafarmaco.it

Agency for Regional Health Care Services
http:///www.assr.it

Netherlands

Netherlands Organisation for Health Research and Development
http://www.zonmw.nl/en/home.html

Health Council of the Netherlands
http://www.gr.nl/index.php

Health Care Insurance Board (CVZ)
http://www.cvz.nl/

New Zealand

Pharmaceutical Management Agency
http://www.pharmac.govt.nz/

Surgical Booking System
http://www.electiveservices.govt.nz

Sweden

Pharmaceutical Benefits Board:
http://www.lfn.se/

Swedish Council on Technology Assessment in Health Care:
http://www.sbu.se/www/index.asp

National Board of Health and Welfare:
http://www.sos.se/sosmenye.htm

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