



BREVE 7

EXPLICIT PRIORITY SETTING IN NEW ZEALAND AND THE UK

Presentation by Tommy Wilkinson. April 2014

A series on policies and methods based on presentations for experts. Prepared by CRITERIA, a knowledge network on prioritization and health benefit plans from the Inter-American Development Bank.



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INTRODUCTION

This *Breve* is based on a webinar presented by Tommy Wilkinson, Health Economics Advisor at NICE International, on April, 2014, to the members of CRITERIA, the Knowledge Network on Health Benefits Packages and Priority Setting in Health.¹

Two prime examples of explicit priority setting in the healthcare space can be found in the United Kingdom and New Zealand. The UK's National Institute for Health and Care Excellence (NICE) and its achievements are recognized around the world. New Zealand's Pharmaceutical Management Agency (PHARMAC) has a similar remit to NICE, in that it determines funded access to pharmaceuticals and other technologies while operating within a range of different constraints and contexts. For many, PHARMAC also represents one of the world's most successful case studies in medical priority setting and pharmaceutical expenditure control.

The presentation and its resulting BREVE provide a perspective on the objectives, capacities and products of NICE² and PHARMAC and explore the comparative advantages of each of these agencies. Specifically, it describes and compares the two agencies and the context in which they work; analyzes and compares their priority-setting processes; and presents an overview of the types of Health Technology Assessment (HTA) conducted. Given the relative lack of information currently available in the literature about processes employed by PHARMAC, this

policy brief will focus on PHARMAC in greater detail. For full information on NICE and PHARMAC, please refer to their websites www.nice.org.uk and www.pharmac.govt.nz, respectively.

COUNTRY CONTEXT: UNITED KINGDOM AND NEW ZEALAND

In order to provide context for the presentation, Table 1 (next page) compares several healthcare indicators between New Zealand and the United Kingdom.

¹ Registered members of the Knowledge Network on Health Benefits Packages and Priority Setting in Health can access the audio and PowerPoint files of the presentation here: <http://www.redcriteria.org/webinars#>

² This policy brief will focus only on the Technology Appraisal program at NICE, which is the program that makes explicit funding recommendations for individual technologies or groups of technologies and is the most easily comparable policy function to PHARMAC. Note that there are many more functions of NICE, such as production of public health guidance and clinical guidelines, quality standards and extensive provision of information for which PHARMAC does not have an equivalent.

Table 1. Comparison of key indicators for New Zealand and the UK

Indicator	New Zealand	United Kingdom
Population, million (2012) ³	4,43	63.7
GNI per capita (2012, USD) ²	\$30,750	\$34,640
Life expectancy at birth ²	81 years	82 years
Organization of the health system	20 District Health Boards, 33 Primary Health Organizations (groups of general practitioners)	In England ⁴ : NHS England providing national leadership and operations, 211 Clinical Commissioning Groups (groups of general practitioners)
Total health expenditure (THE, US\$) ²	US\$12.5 billion (2012)	US\$202 billion (2011)
Per capita health expenditure (PPP constant 2005 international \$, 2012) ²	3,292	3,495
THE, as % GDP ²	10%	9%
Public expenditure on health, as % of THE ²	83%	83%
Gini coefficient ²	0.36	0.34
Expenditure on medicines per capita (2009) ⁵	US\$265	US\$380

New Zealand (NZ) and the United Kingdom (UK) share very similar health profiles. Although the UK has a much larger population, the GNI per capita and life expectancy at birth are both very similar, as well as the two countries' total health expenditure as a percentage of GDP and per capita health expenditure.

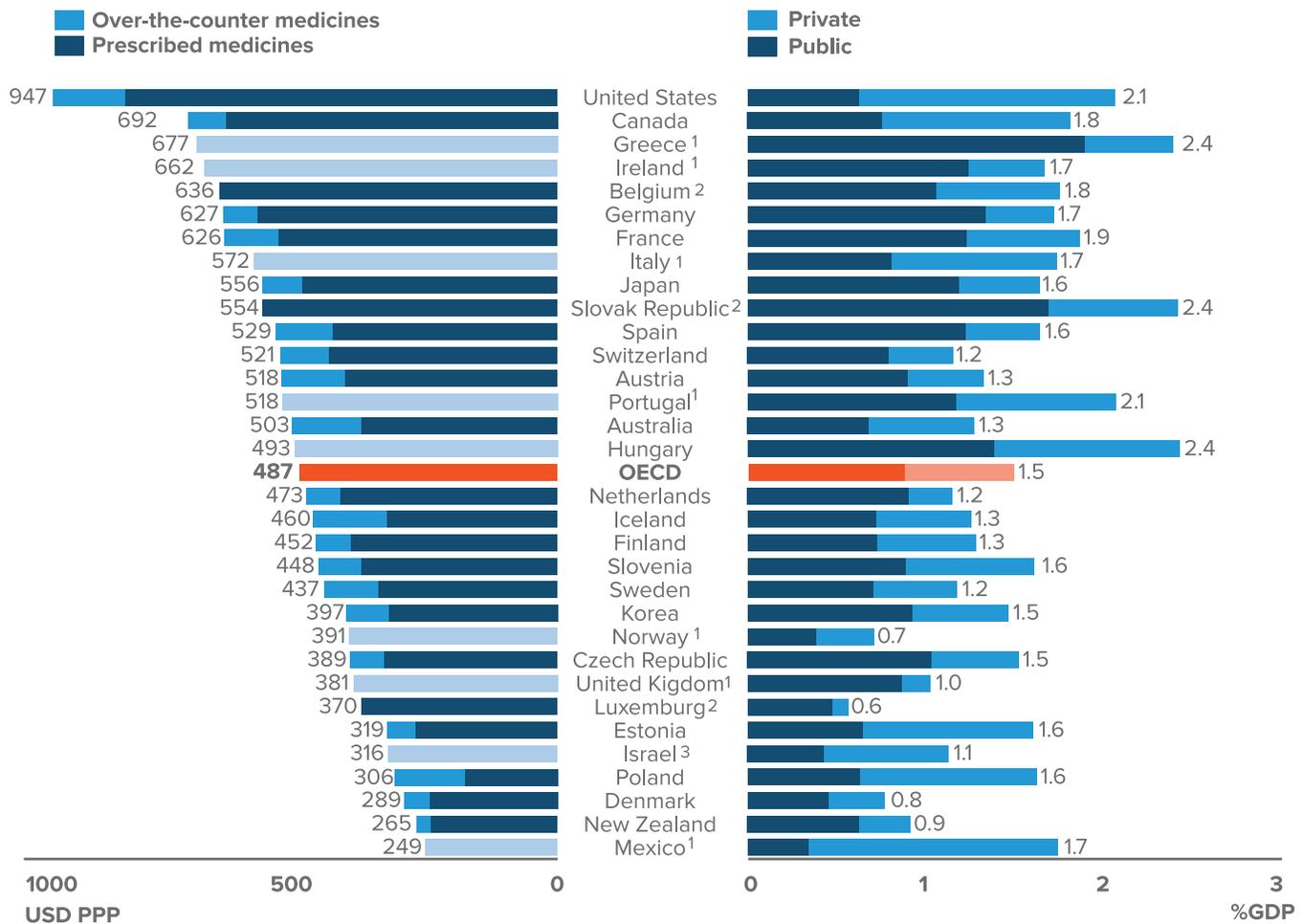
Compared to other OECD countries, their pharmaceutical expenditure is low, at US\$380 in the UK and US\$265 per capita in NZ, or roughly 1% of GDP in both countries. As shown in figure 1, pharmaceutical expenditure in NZ in terms of total amount and proportion of GDP is among the lowest in the OECD, which can be largely attributed to the work of PHARMAC.

³ <http://databank.worldbank.org>

⁴ Only organization of health system in England described. For further information on organizations of health system across the UK, see <http://www.nao.org.uk/report/healthcare-across-the-uk-a-comparison-of-the-nhs-in-england-scotland-wales-and-northern-ireland/>

⁵ OECD (2011), "Pharmaceutical expenditure," in *Health at a Glance 2011: OECD Indicators*, OECD Publishing.

Figure 1. OECD country drug spending per capita and as a share of GDP (2009)



1 Cannot be separated and includes medical non-durables
 2 Prescribed medicines only
 3 Total medical goods
 Source: OECD Health Data 2011

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE (NICE): AN OVERVIEW

Before NICE existed, the quality of the UK health system varied widely. The adoption of newer standards of care and technologies was often slow, and out-of-date practices persisted. As a result, the UK population faced a significant variation in the quality of care they received depending on where they lived, a phenomenon known as the “postcode lottery.” Given these pressures, in 1999, the UK government established the National Institute for Health and Care Excellence (NICE)⁶.

⁶ Then known as the National Institute for Clinical Excellence

NICE is expected to (i) serve as a national, authoritative source of advice; (ii) provide guidance based on effectiveness and cost-effectiveness; (iii) use an inclusive and consultative approach that incorporates views of the larger society; (iv) be efficient and independent of patient, political or industry influence; and (v) respond to social values and the needs of the public. As a result, the agency has gained broad support from professional groups, patient groups and the general public.

Today, the functions of NICE include:

1. The production and dissemination of guidance for individual technologies, clinical guidelines, public health, and social care.
2. The development of performance standards and metrics for overseeing the implementation of these guidelines.
3. The provision of general information to health professionals and patient groups to empower them to make their own healthcare decisions.

The NICE decision process shown in Figure 2, which permeates all aspects of NICE, demonstrates the cyclical nature of how decisions are made within the organization. The agency looks at new evidence, appraises it, consults with stakeholders, produces guidance, and updates the decisions. The institute continuously repeats the cycle by seeking new evidence.

Figure 2. NICE’s decision cycle

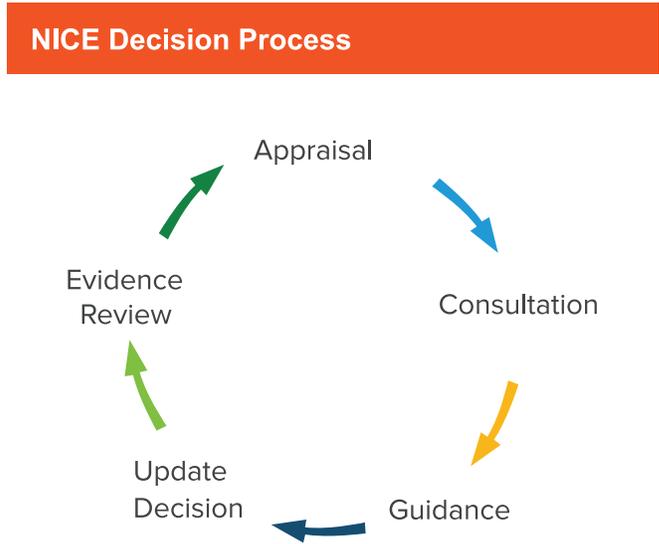


Figure 3. NICE’s organizational structure⁷



Organizational structure correct as of April 2014

⁷ Organizational structure correct as of April 2014

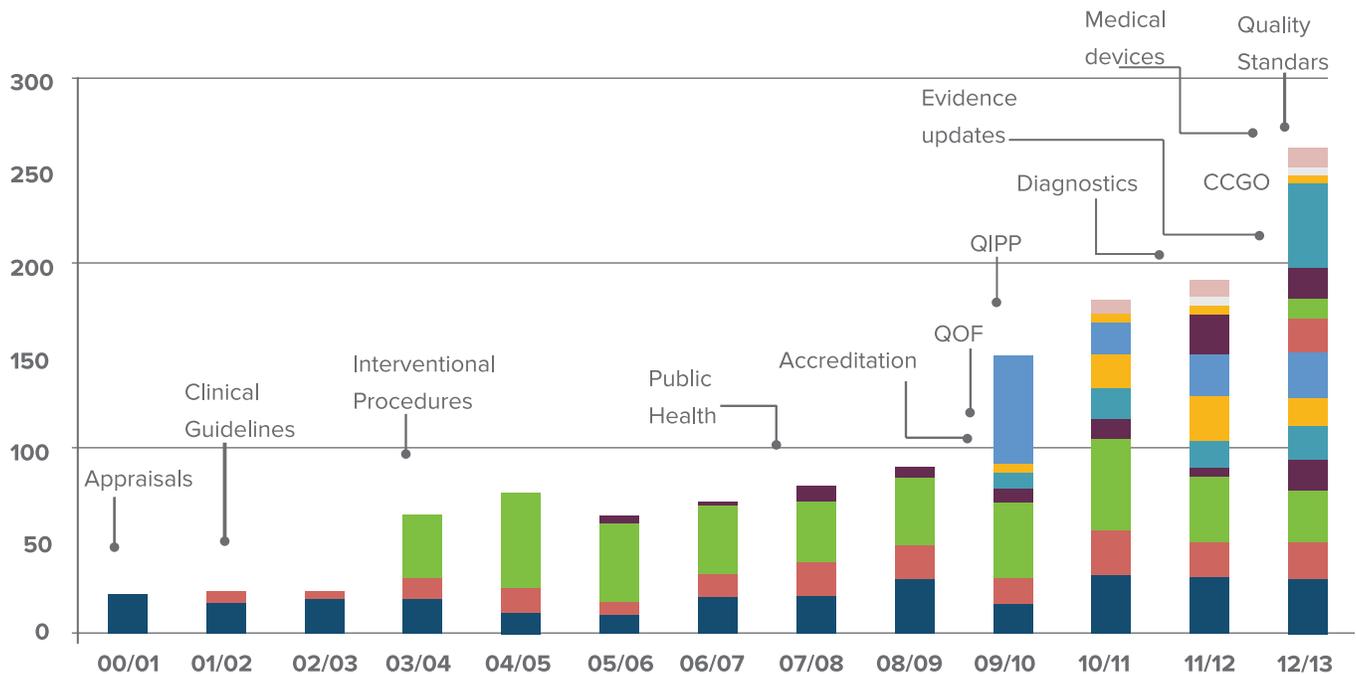
NICE is a fairly large organization with approximately 550 staff members divided into eight directorates. The most well-known agency is the Center for Health Technology Evaluation, which receives international media attention for its drug and technology evaluations. The Health and Social Care Directorate provides guidance on how social care services should operate.

The Center for Clinical Practice produces clinical guidelines, and the Evidence Resources Directorate manages a library of evidence, conducts systematic reviews of clinical evidence and provides summaries

to internal and external stakeholders. The other directorates, including the Business, Planning, Resources and the Communications Directorate, are also essential to the successful operation of NICE within the National Health Service.

NICE produces a wide range of outputs: appraisals of drugs, medical technologies, devices, and diagnostics; a library of clinical evidence; quality standards; public health guidance; clinical practice guidelines; recommendations on staffing; and social care guidance. Outputs have increased in number and variety with each year since the establishment of NICE, as shown in figure 4.

Figure 4. Products of NICE, by year

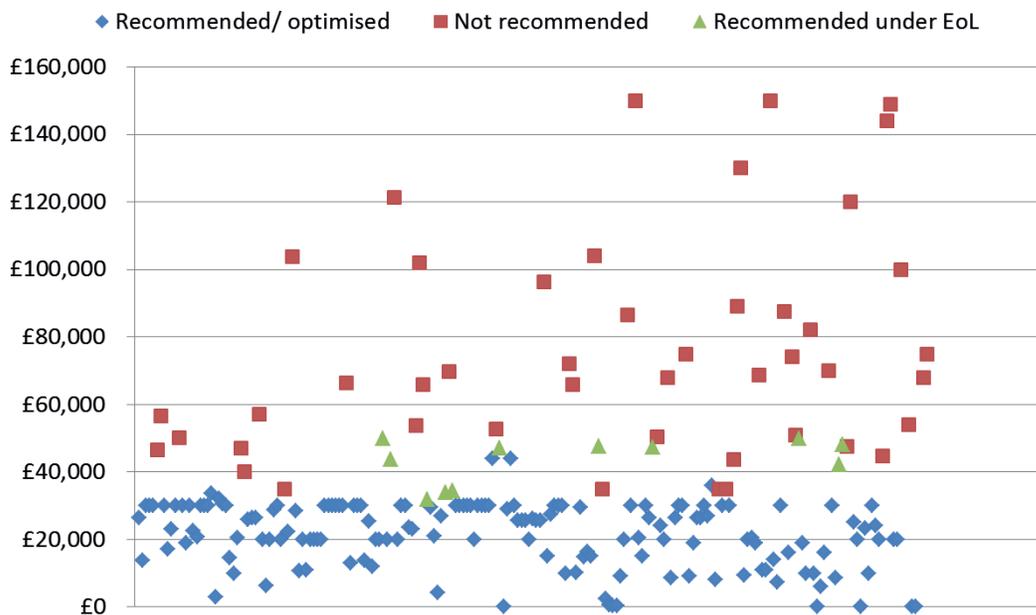


SOURCE: NICE INTERNATIONAL

Figure 5 shows the most plausible estimate of the incremental cost effectiveness ratio (ICER) of specific individual technologies appraised by NICE. The ICER is a measure of efficiency of a health technology and estimates the cost (measured in British pounds) required to achieve an amount of health gain (measured in quality-adjusted life years [QALY])⁸. Each individual point in figure 5 represents a decision by NICE, where “a recommend” is represented in blue and green⁹, and decisions not to recommended technologies are represented in red. Most of the approved technologies cluster at or below £30,000 per QALY, reflecting the threshold range used to inform recommendations. For technologies above a most plausible ICER of £30,000 per QALY, NICE’s Appraisal Committees will need to identify an increasingly stronger case for

supporting a technology as an effective use of NHS resources¹⁰. It is important to note that while the estimate of the ICER informs recommendations by NICE (through their Appraisal Committees), the ICER is not the only consideration taken into account by NICE when deciding whether to recommend a technology. Considerations beyond efficiency, such as the need to distribute resources fairly and equity considerations, also play a role¹¹, in addition to innovation, the uncertainty in the analysis, and whether there may be health gain from the health technology that has not been captured in the analysis. These additional factors are considered qualitatively by NICE’s Appraisal Committees when deciding whether to recommend a technology for use in the National Health Service.

Figure 5. Most plausible ICER for technologies appraised by NICE, 2007–Sept. 2013



SOURCE: NICE INTERNATIONAL

⁸ See <http://www.nice.org.uk/glossary> for more information on the QALY and ICER and how they are used by NICE.

⁹ Green points represent technologies approved under the end-of-life premium. See NICE Guide to Methods of Technology Appraisal.

¹⁰ See NICE Guide to the Methods of Technology Appraisal at <http://www.nice.org.uk/article/pmg9/chapter/foreword>

¹¹ <http://www.nice.org.uk/media/SVJ2PUBLICATION2008.pdf>

PHARMACEUTICAL MANAGEMENT AGENCY (PHARMAC): AN OVERVIEW

New Zealand's analogous agency, PHARMAC, is an independent crown agent. Although funded by the Ministry of Health, it operates independently and reports directly to the Minister of Health. In New Zealand during the 1980s, expenditure on pharmaceuticals was increasing at a faster rate than other healthcare spending and was one of the fastest growing items of government expenditure. Growth of more than 20% in some years meant pharmaceutical expenditure was threatening to crowd out other healthcare funding. This led to the creation of the Pharmaceutical Management Agency (PHARMAC) in 1993 to actively manage government spending on medicines that, at the time, amounted to \$445 million¹². PHARMAC currently operates with approximately 110 full- and part-time staff and, in 2014, manages an annual primary care pharmaceutical budget of NZ\$795 million (US\$603 million).

The legislative function of PHARMAC is defined by the New Zealand Health and Disability Act 2000 as the following: "To secure for eligible people in need of pharmaceuticals, the best health outcomes that can reasonably be achieved, and from within the amount of funding provided." The key feature of the law that sets PHARMAC apart from other agencies is the explicit budgetary limitation.

PHARMAC operates by nominally accepting budget responsibility for a portion of each District Health Board's budget to be used for pharmaceutical expenditure (usually 7-8% of its annual budget). PHARMAC uses this budget constraint to decide which medicines will be funded by the government. The agency regularly produces a list of government-funded community and hospital medications, which indicates the brands of funded substances and, in some instances, their restricted indications. This list is called the Pharmaceutical Schedule, and it has a major influence on prescribing and dispensing behavior in the New Zealand health system. The agency also operates a number of demand-side activities to encourage appropriate and safe use of pharmaceuticals and performs linked functions such as special panels to determine funding for pharmaceuticals in exceptional circumstances.

PHARMAC has seen remarkable success in controlling medical expenditures, and its scope of operations has increased since 1993. In 1997, PHARMAC conducted the first tender for sole supply of community pharmaceuticals, a competitive process that awards funded access to a single supplier of an individual medicine. In 2002, PHARMAC was given the responsibility for managing all medicines used in the treatment of cancer, both those used within and outside of the hospital. This included determining which medicines would be funded by the government and defining the terms of the contract with pharmaceutical companies. In 2003, the agreed annual spending on all community pharmaceuticals increased to \$510 million. By that point, PHARMAC claimed a cumulative savings to New Zealand of \$2 billion over the first decade of its operation, which is even more remarkable when taking into account that prescription volume had grown at an annual rate of 6% during the same period. Another

¹² Taken from <http://www.pharmac.health.nz/about/our-history/>

decade later, in 2012, PHARMAC was asked to manage the purchasing of vaccines. In the second decade of its operations, PHARMAC helped New Zealand save an additional \$2 billion in drug expenditure.

One of the key tools PHARMAC has to control spending is its sole supply tender process. The prospect of 100% market share, without the need for marketing or a sales force, for a defined period of time (usually three years) is extremely attractive to potential suppliers to the New Zealand market. The size of New Zealand is an advantage in this regard. In the tender documentation, PHARMAC would indicate to potential suppliers the

expected size of a particular pharmaceutical market, which often enabled international manufacturers with large operations to easily supply the entire national market without substantial outlay. The market certainty offered through the tender process would often result in significant price reductions relative to international norms.

The graph in figure 6 demonstrates the significant savings PHARMAC has achieved in its relatively brief history. The orange line represents what was actually spent on medicines since 2000, and the blue line represents what would have been spent in the absence of PHARMAC, which is not dissimilar to drug spending in many other countries. An example of just how much price reduction can be achieved through sole supply negotiations carried out by PHARMAC is provided in figure 7.

Figure 6. Impact of PHARMAC – Actual spending vs. estimated spending in primary care (community pharmacy)¹³

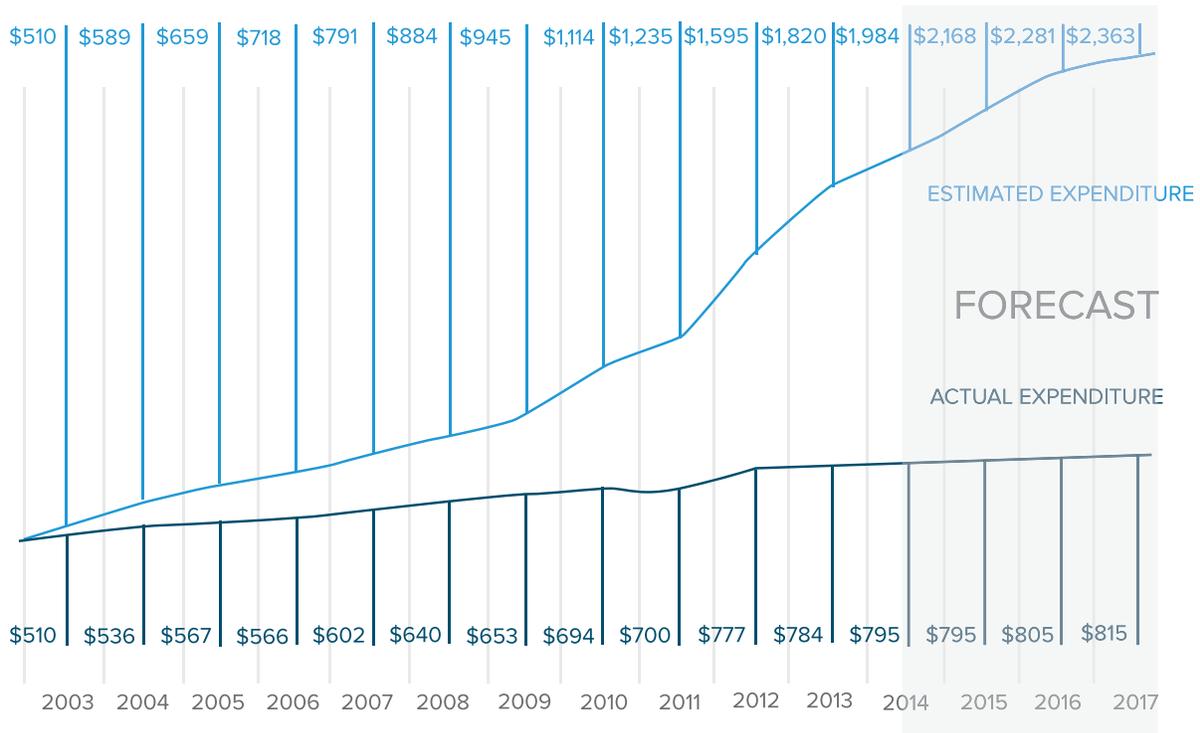
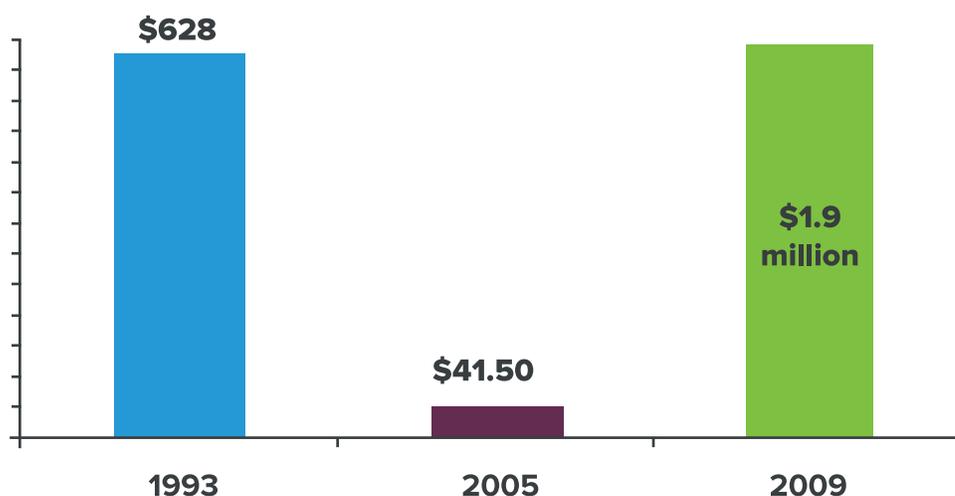


Figure 7. Case Study #1: Price reduction achieved with Fluconazole 200mg capsules



In 1993, PHARMAC was paying NZ\$628 for a 200 mg pack of capsules of Fluconazole. In the first tender conducted in 2005, PHARMAC was able to reduce the price by 93% to \$41.50 in exchange for three years of sole supply rights for all of New Zealand to one pharmaceutical manufacturer. At the second tender in 2009, PHARMAC achieved a further 68% reduction, resulting in a price of \$13.34 per pack. It is estimated that, per year, \$2 million was saved on this single formulation, using a simple tender without any need for negotiation, and it enabled new investments in pharmaceuticals. While this achievement required sound management, a consolidated approach and political support, it was clearly very successful at managing pharmaceutical expenditure.

“DECISION TO FUND” PROCESS AT PHARMAC

PHARMAC’s decision to fund a treatment (i.e., to list it on the Pharmaceutical Schedule) follows these steps:

Step 1: Receipt of proposals from the public, which can include anyone from health professionals to pharmaceutical companies to patient groups

Step 2: Medical Advice – The Pharmacology and Therapeutics Advisory Committee (PTAC) is the primary clinical advisory committee, whose role is to consider clinical evidence and provide objective advice to the

Board of PHARMAC. PTAC is comprised of senior health practitioners from a range of specialties who consider both clinical evidence and PHARMAC’s nine decision criteria before making recommendations (see section below).

Step 3: Economic assessment – assesses the relative value of the drug

Step 4: Prioritization for funding

Step 5: Negotiation

Step 6: Consultation

Step 7: Decision

Step 8: Implementation

The process is often nonlinear. For example, if prioritization for funding is low due to cost (step 4), but negotiation reduces the price significantly (step

5), PHARMAC goes back to step 3 and its economic assessment improves. This change moves the treatment to a higher priority (step 4), thereby enabling the drug to continue through to consultation (step 6) and ultimately implementation (step 8). Economic assessment (step 3), decision-making (step 7), and pricing strategies are explained in greater detail below.

ECONOMIC ASSESSMENT AT PHARMAC (STEP 3)

1. Pharmacoeconomic analysis. PHARMAC’s Prescription for Pharmacoeconomic¹⁴ Analysis outlines the specific methods of Health Technology Assessment (HTA) used by staff and in submissions to PHARMAC. Depending on the decision that needs to be made, PHARMAC employs various levels of HTA as listed in table 2. In general, detailed analyses take a significant amount of time and most closely resemble the HTA methods used by NICE. PHARMAC has developed other, more agile types of HTA methods to enable the agency to take advantage of certain spending opportunities. For example, the rapid assessment typically takes less than two weeks and can be performed by one analyst.

Table 2. HTA methods used by PHARMAC¹⁵

TYPE OF HTA	DESCRIPTION	TIME REQUIRED
Detailed	<ul style="list-style-type: none"> • Includes a detailed and systematic identification and synthesis of relative clinical effectiveness, prognosis, health-related quality of life (HRQoL), and cost data. • Evidence critically appraised using the Graphic Appraisal Tool for Epidemiology (GATE) framework (or other similar tools). • Detailed Markov model. All potential health states and clinical events included. The use of probability distributions considered. • Detailed extrapolation of the clinical evidence and statistically non-significant events tested. • Further validation of utility mapping exercise, including obtaining expert clinical input. • Probabilistic sensitivity analysis may be undertaken. • Reviewed internally and externally (clinical assumptions reviewed by Pharmacology and Therapeutics Advisory Committee (PTAC)). 	>2 months

¹⁴ <http://www.pharmac.health.nz/assets/pfpa-final.pdf>

¹⁵ <http://www.pharmac.health.nz/assets/pfpa-final.pdf>

Indicative	<ul style="list-style-type: none"> • An interim assessment using some opportunistic data, but more detailed than a preliminary analysis. Evidence critically appraised. • Often involves more complex economic modelling. Full assessment undertaken on whether statistically insignificant events are likely to be clinically significant. • Further investigation into HRQoL scores, including a systematic review of the literature. • Full multivariate sensitivity analysis may be undertaken with detailed discussion of results. • Detailed documentation of critical appraisal and economic analysis. • Reviewed internally and by PTAC. 	1-2 months
Preliminary	<ul style="list-style-type: none"> • Assessment largely using opportunistic data. • Rapid systematic review of evidence undertaken. • May require further modelling compared with a rapid CUA (due to disease complexity, risk, or uncertainty of results). • Reviewed internally. 	2-4 weeks
Rapid	<ul style="list-style-type: none"> • Basic economic model constructed, largely based on opportunistic data. The analysis is undertaken over a time horizon that sufficiently captures the majority of incremental costs and benefits. • Testing undertaken to ensure extent of analysis is sufficient. • Brief documentation of CUA (but still detailed enough to allow reproduction of the CUA by others). • Reviewed internally. • May include reviews and basic amendments to external analyses. 	<2 weeks

2. Budget analysis – an analysis of the cost of the medicine with respect to the budget. This analysis shows the costs or savings to the sector as a whole (e.g., hospital resources). What makes PHARMAC unique (and distinct from NICE) is that the agency runs both a

budget analysis and a cost utility analysis together to make its decisions.

PHARMAC DECISION- MAKING CRITERIA (STEP 7)

Originally defined by the Ministry of Health, PHARMAC uses the following nine decision criteria when making recommendations:

1. The health needs of all eligible people within New Zealand;
2. The particular health needs of Maori and Pacific peoples¹⁶
3. The availability and suitability of existing medicines, therapeutic medical devices and related products and related things;
4. The clinical benefits and risks of pharmaceuticals;
5. The cost-effectiveness of meeting health needs by funding pharmaceuticals rather than using other publicly-funded health and disability support services;
6. The budgetary impact (in terms of the pharmaceutical budget and the government's overall health budget) of any changes to the Schedule;
7. The direct cost to health service users;
8. The government's priorities for health funding, as set out in any objectives notified by the Crown

¹⁶ This criterion refers to obligations of the New Zealand government relating to the Treaty of Waitangi. The Treaty of Waitangi is a written agreement signed between more than 500 Maori chiefs and the British Crown in 1840, which led to formal colonization of New Zealand by Britain. One specific example where the decision-making criteria relating to the particular needs of Maori and Pacific peoples was considered relevant was a decision regarding the drug trastuzumab in the treatment of early breast cancer. Although Maori women had far higher rates of breast-cancer associated mortality, high proportions of late-stage presentation made it uncertain whether making trastuzumab available in early breast cancer would have a substantial impact on mortality in Maori women. If the opportunity cost of funding trastuzumab fell on resources for early detection and awareness of breast cancer (which was entirely possible), funding trastuzumab could have resulted in worse outcomes for Maori women overall. PHARMAC initially declined funding for trastuzumab referring to this criterion, and ironically, several Maori interest groups were actively in favor of approving trastuzumab in early breast cancer, citing the high breast-cancer associated mortality in Maori women. See <http://www.pharmac.govt.nz/2007/06/13/290607c.pdf#text> for more information.

to PHARMAC, or in PHARMAC's Funding Agreement, or elsewhere; and

9. Such other criteria as PHARMAC thinks fit. PHARMAC will carry out appropriate consultation when it intends to take any such "other criteria" into account.

Despite early criticism about redundancies, PHARMAC has successfully¹⁷ operated with these criteria by applying them in a pragmatic and transparent manner. A key differentiator between these criteria and those of NICE is how these criteria expand beyond cost-effectiveness and explicitly take budgetary impact into account. While NICE will consider factors beyond cost effectiveness (efficiency) in its decision-making, PHARMAC's approach allows for more flexibility.

It is also important to highlight that in addition to these criteria used during the decision-making process, PHARMAC has an exceptional circumstances process that may provide funding on an individual patient basis. Criteria for exceptional circumstances funding include:

- Rare disease - affects a very small number of patients (in NZ it would be 10 cases or fewer),
- Severity of disease
- Availability of alternatives

An Exceptional Circumstances Panel can convene urgently, within 24 hours' notice, and consider individual patients' cases. The panel meets once a week by teleconference and decides on individual applications (in England, this function is performed at a local Clinical Commissioning Group level, a process that does not involve NICE).

¹⁷ Given the lack of an alternative pharmaceutical policy in New Zealand against which to compare PHARMAC, it is difficult to empirically and definitively assert "success." Nevertheless, the continued operation of PHARMAC for more than 20 years under successive governments with changing priorities, its continued expansion and remit, and successful defense of methods and process within the New Zealand judicial system indicates some level of success.

In determining whether a treatment is considered to fall under “exceptional circumstances,” PHARMAC must first review how many patients have been affected. Some of those interventions would be classified as usual pharmaceuticals, while others would fall under “orphan drug” criteria. After this designation, PHARMAC makes a decision by relying on its nine decision criteria. In theory, the agency could assess and approve an orphan drug that was extremely cost-ineffective because of the unavailability of other drugs for that condition.

PRICING STRATEGIES

PHARMAC relies on the following strategies to reduce and manage drug prices:

1. Tendering – used for off-patent drugs; nearly a third of the 1,700 formulations listed on the Pharmaceutical Schedule are sourced through tendering. The tender process is one of the strongest tools PHARMAC has for purchasing generic drugs.
2. Caps and rebates – confidential pricing and supply arrangements negotiated with suppliers and manufacturers; PHARMAC possesses substantial negotiating power.
3. Therapeutic reference pricing – all pharmaceuticals in any given therapeutic sub-

group to which PHARMAC decides to apply reference pricing are subsidized at the level of the lowest-priced pharmaceutical (usually a generic) in that sub-group. For example, reference pricing has been applied to the statin therapeutic sub-group in the past, where funding for all statins was set at a level of the lowest therapeutic equivalent dose of simvastatin, and the additional charge (copayment) is borne by patients if they do not want to switch to a fully-funded alternative. On occasion, therapeutic reference pricing is combined with Special Authority (see below) to fund the copayment for patients who cannot tolerate the referenced pharmaceutical. Although this mechanism has been successful in the past, it also generates significant objections from the pharmaceutical industry.

4. Subsidy by special authority – control of specific patients that can receive funding for certain drugs (see the case study “Special Authority Mechanism”). While NICE might recommend that the use of a particular technology in the NHS be optimized (i.e., used only in a restricted patient population for whom the use of the technology is cost-effective), compliance with the optimized recommendation is managed at a local commissioning group or hospital level, with relatively limited powers to enforce prescribing compliance (figure 8)

Figure 8. Special authority mechanism

Candesartan

INITIAL APPLICATION

Applications from any relevant practitioner. Approvals valid without further renewal notified.

Prerequisites (tick boxes where appropriate)

Patient with congestive heart failure

and

Has been treated with, and cannot tolerate, two ACE inhibitors, due to persistent cough
or

Has experienced angioedema on an ACE inhibitor at any time in the past or who have experienced angioedema (even if not using an ACE inhibitor) in the last 2 years

or

Patient with raised blood pressure

and

Use of fully funded beta blockers or diuretics are contraindicated; or not well tolerated; or insufficient to control blood pressure adequately at appropriate doses

and

Has been treated with, and cannot tolerate, two ACE inhibitors, due to persistent cough
or

Has experienced angioedema on an ACE inhibitor at any time in the past or who have experienced angioedema (even if not using an ACE inhibitor) in the last 2 years

Special Authority Mechanism

PHARMAX'S special authority mechanism enables it to finance a particular drug only if the patient fulfills specific criteria. The request for approval can be filed electronically by the physician at the point of prescribing, generating an approval number (often within less than a minute), which enables patients to fill the prescription in a pharmacy. The length and detail of a Special Authority application varies. Some Special Authority applications are very simple, such as pioglitazone, which requires ticking just one box. In contrast, the application for sildenafil for pulmonary hypertension requires panel approval and is 27 pages long. It is also worth noting that this authority mechanism restricts funding just to the particular indication for which the drug was approved. NICE does not have a similar mechanism in place.

COMPARISON OF THE HTA PROCESSES AT PHARMAC AND NICE

PHARMAC and NICE are both national agencies that consider clinical and cost-effectiveness in addition to social value judgments when making decisions that result in funded access to health technologies. Although the methods for conducting a health technology assessment¹⁸ are very similar at PHARMAC and the Technology Appraisals program at NICE, the main departure between the two agencies is the way that the evidence produced through HTA is used in relation to the budget constraint. PHARMAC engages in HTA-informed proactive procurement and negotiation under an explicitly-stated budget constraint, whereas NICE uses a cost-effectiveness threshold as the primary decision rule¹⁹, where the threshold is estimated to reflect the opportunity cost resulting from the budget constraint. This difference is largely a function of how the opportunity cost of funding decisions is recognized. PHARMAC has a predefined annual budget, enforcing pragmatic consideration of the opportunity cost of investment decisions. NICE does not have responsibility for pharmaceutical expenditure, and so is primarily concerned with determining the value for money of a technology.

Another important difference is related to the different parties conducting and managing the process, which has implications for independence and transparency. PHARMAC conducts all HTA and negotiations in-house, limiting the real-time involvement of patient representatives and other stakeholders in the process, except during the general consultation process. NICE is highly transparent, providing comprehensive consultation and engaging in active public involvement. In addition, the use of independent Appraisal Committees made up of practicing health professionals, academics, technical specialists and patient representatives provides another layer of transparency and wider stakeholder involvement. The value of differing levels of transparency is beyond the scope of this policy brief, and it is highly likely that PHARMAC would not be able to achieve such significant price reductions for pharmaceuticals if it were as transparent as NICE.

Tables 3 and 4 below compare and contrast various aspects of the agencies and their processes.

¹⁸ Some of the key methodological experts who were involved in developing NICE HTA methods were also involved in the development of PHARMAC HTA methods.

¹⁹ As described earlier NICE's Appraisal Committees also take into account "considerations beyond efficiency" when making recommendations

Table 3. Comparison of HTA processes at PHARMAC and technology appraisals at NICE

	PHARMAC	NICE
Topic selection	Initial in-house topic prioritization process. Submission by manufacturer must meet guidelines (information on clinical benefits and possible costs) and requires a pre-submission meeting	Horizon scanning, scoping, referral by Minister of Health, submission by manufacturer/industry
Who does HTA	Carried out in-house (by PHARMAC staff) using one of several methods as defined in the Prescription for Pharmacoeconomic Analysis ²⁰	Manufacturer with review/ quality assurance by Independent Academic Group (IAG) (alternatively IAG conducts HTA de novo). ²¹ All HTA conducted in line with Methods of Technology Appraisal ²²
Who evaluates evidence	PTAC (+/- subcommittee) reviews evidence and makes recommendation; minutes are published; prioritization exercise; contract negotiation; consultation	Appraisal Committee with support of Independent Academic Group reviews evidence and makes recommendation for consultation, with possibility of appeal after final recommendation
Who gives final approval for funding directive	PHARMAC Board	Appraisal Committee (this function has been devolved from the Secretary of State for Health)
Process outcome	Communication/ implementation	Communication/ implementation, followed by review

²⁰ <http://www.pharmac.health.nz/assets/pfpa-final.pdf>

²¹ De novo HTA is conducted by Independent Assessment Groups under the multiple technology Appraisal Process

²² <http://www.nice.org.uk/article/pmg9/chapter/foreword>

Table 4. Comparison of agency features

Characteristic	PHARMAC	NICE
Role of budget in decision	Responsibility to limit spending within the given budget	No responsibility to maintain or limit pharmaceutical spending (explicitly will not look at the budget in making a decision)
Decision criteria	stated decision criteria	criteria, but this is applied judiciously while taking considerations beyond efficiency into account
Roles in pricing, procurement and supply of pharmaceuticals	commercial activities: contract management, tenders and negotiations; wide remit around the supply of pharmaceuticals; determines dispensing rules for pharmacists	Limited. NICE does manage the Patient Access Scheme process, which facilitates price negotiation between the Department of Health and manufacturers for some technologies. Initiatives for procurement and supply of pharmaceuticals is conducted at a sub-national and local level
Source of health economics advice	Manufacturer will make submission which includes evidence of cost-effectiveness. Health economics advice is in-house	Manufacturer will submit application. Independent Academic Group will review. Appraisal conducted by independent Committee with secretariat support from NICE staff.
Flexibility of process	Flexible process, with reference to what can be done and what can be deal	Operates within defined and consistent process only
Funded medicines list	PHARMAC manages the Pharmaceutical Schedule, which constitutes a specific list of approved medications (correlates to budget)	No positive list of funded pharmaceuticals. While NICE does recommend which medicines should and should not be funded, a large proportion of pharmaceuticals currently in funded use have not and will not be evaluated under NICE's Technology Appraisal program.
HTA methodology	Varying degrees of complexity of HTA methods for each decision	The same in-depth process followed for every technology evaluated under the Technology Appraisal process ²³

23 Standardized process followed depending on whether technology is to be assessed with other similar technologies (Multiple Technology Appraisal) or individually (Single Technology Appraisal)

REFLECTION: WOULD YOU CHOOSE PHARMAC OR NICE FOR YOUR COUNTRY?

There are both advantages and disadvantages to the PHARMAC and NICE approach:

- A. NICE – a robust, transparent, procedure-based organization based on sound social and health economic theory; seeks to achieve allocative and technological efficiency through the use of an estimation of the threshold as the decision rule
- B. PHARMAC – pragmatic, flexible yet less transparent process but has proven ability to contain and constrain spending while maintaining access to pharmaceuticals

Nonetheless, both types of organizations are based on these key non-negotiables:

- i. Transparency, clarity, and consistency of process
- ii. Independence of organization
- iii. Clear decision rules that are transparent and could be reviewed if amendments are needed

It is important to note that NICE does offer a much wider range of outputs compared to PHARMAC. The production of clinical guidelines and quality standards in particular have a major influence on practice in England,

and they provide a mechanism for resource allocation decisions to be incorporated into comprehensive guidance and implementation support to encourage best practice.

Some aspects of the way that PHARMAC and NICE operate are simply a reflection of their population size and political context. For instance, the pharmaceutical industry is not a major employer in New Zealand, which frees it from having to incorporate industrial policy into health resource allocation decisions. In addition, it is likely that competitive pricing strategies such as national sole supply tender would be unworkable in England given the large patient populations and European Union trade and import regulations. Many of PHARMAC's functions such as making exceptional circumstances funding decisions, procurement, and medicine use optimization initiatives are done in England but on a much more localized level, making a direct comparison of many of the NICE/PHARMAC functions difficult.

Therefore, a key consideration for countries wanting to learn from the experiences of PHARMAC and NICE is whether the common and successful features of pharmaceutical policy conducted in both New Zealand and England should be conducted at a local or national level. This is likely to reflect country size, the nature of health financing, and resource allocation decisions. to conduct HTA, but decision-makers refer to this one entity for their information needs. Other countries stated that purchasers might also perform HTA, which could mean regions or provinces in decentralized countries, or individual health insurance funds.

Nevertheless, the fundamental concept that indirectly or directly influences many of the differences between PHARMAC and NICE is the explicit consideration of a set

budget by PHARMAC and the estimate of opportunity cost used by NICE. The limitation for PHARMAC in using a predefined budget is that this budget relates only to community pharmaceuticals; therefore, the trade-off will always be the health that could be achieved through the use of pharmaceuticals. Consequently, the concern is that if a pharmaceutical was available that offered substantially better health gain per dollar (i.e., was more technically efficient) than other means of generating health in the New Zealand health service, it might not be funded if its budgetary impact was too high, resulting in sub-optimal allocation of resources and lower overall health from the funding available. In addition, it is also possible that a pharmaceutical might be approved for funding, but due to the limited budget, the funding would not start until the following financial year. The fairness and political tolerability of allowing budget cycles to dictate people's access to a pharmaceutical that could potentially make a substantial impact on length and quality of life need to be considered; however, one could argue that these are concerns in theory only, and that the nature of health policy decision-making, the ability to specify exceptions to the rule, and annual budget-setting means that the system could incrementally and pragmatically self-correct. In addition, the sub-optimality in applying a fixed budget may simply be a reflection of reality. By acknowledging that no decision-making process will ever be able to achieve perfect allocative efficiency, there is a strong argument that making the decision-maker explicitly responsible to both the patient and the tax payer is the only way to achieve optimal allocation of health resources.

Applying a cost-effectiveness threshold to estimate the opportunity cost of investments as used by NICE theoretically addresses the limitations of budget cycles

and siloed budgets, and allows NICE to generate consistent recommendations across technology types for preventative, curative and palliative care for all areas of the NHS. This approach is both more politically acceptable and founded in coherent economic theory, as a well-calibrated threshold will reflect the opportunity cost caused by the budget constraint (for more information on the NICE cost effectiveness threshold see McCabe et al., 2008²⁴). Rightly or wrongly, however, the use of an explicit threshold (or threshold range) does place substantial focus on HTA methods used to estimate a technology's incremental cost effectiveness ratio and whether the threshold used has been calibrated properly. Recent work by Claxton et al.²⁵ indicates that the threshold currently used by NICE may indeed be too high—that is, it underestimates the opportunity cost of resource allocation as a result of NICE guidance.

There is no right answer as to which system would be most appropriate for a particular country. While decisions about the HTA methods and processes that a national HTA agency should employ might appear simply technical and where one can follow “best practice,” they have profound ethical, social and practical implications; therefore, these are decisions for countries themselves to make, drawing on (but not necessarily imitating) the experiences of other countries.

Importantly, both NICE and PHARMAC have been operating for many years and are viewed as relatively successful in their respective countries. Each has been able to do this by engaging with the public and policy makers, continuing to improve

24 McCabe, C., Claxton, K., and Culyer A., *Pharmacoeconomics* 2008; 26 (9): 733-744

25 Methods for the Estimation of the NICE cost-effectiveness threshold. Centre for Health Economics, University of York. June 2013, http://www.york.ac.uk/media/che/documents/reports/resubmitted_report.pdf

and update methods and processes, and providing a useful service for their countries in line with their stated objectives. It is hoped that lessons can be learned from both PHARMAC and NICE to improve locally relevant, acceptable, and sustainable priority setting in countries around the world.

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