

PHARMAC's Decision Criteria Consultation document

May 2013



PHARMAC's Decision Criteria Review

Message from Steffan Crausaz, Chief Executive, PHARMAC

PHARMAC's Operating Policies and Procedures (OPPs) outline how we carry out our core statutory obligations: they guide how we do what we do. For the people and organisations we work with, our OPPs are a tool for working with us, and they function as a guidebook for PHARMAC staff when considering pharmaceutical funding proposals, managing the Pharmaceutical Schedule and making changes to our processes and other work.

PHARMAC's OPPs include our nine decision criteria for pharmaceutical funding. These criteria are central to our prioritisation and funding decision-making, which in turn directly affect the pharmaceutical treatments that are available for public subsidy in New Zealand. And, as PHARMAC moves towards managing the funding of medicines and medical devices used in District Health Board hospitals, we need to take stock of our existing processes to make sure that they are still fit for purpose.

PHARMAC's work touches most New Zealanders in some way. Every time you, or someone you know, take a prescription to your local pharmacy you are impacted by the decisions PHARMAC makes. Having your say on the criteria we use to help us make those decisions should mean our funding decisions continue to reflect the things New Zealanders – the public who use subsidised pharmaceuticals, those who prescribe them and the industry that makes them – value.

I hope you take this opportunity to help shape the way PHARMAC makes its decisions and I look forward to reviewing your feedback.

Steffan Crausaz
Chief Executive

Contents of this consultation document

1. Introduction
 2. Content and review process
 - 2.1 Origins and aims
 - 2.2 Process to date
 - 2.3 Seeking your feedback
 - 2.4 Submitting your response
 - 2.5 Next steps
 3. Professor Anthony Harris' discussion paper – *On what basis should we decide about health care priorities?* – executive summary
 4. Background
 - 4.1 Introduction to PHARMAC
 5. PHARMAC's decision criteria
 - 5.1 The purpose of the decision criteria
 - 5.2 When and how PHARMAC uses the decision criteria
 - 5.3 Medical devices
 - 5.4 Other considerations
- Appendix 1: Guiding questions
- Appendix 2: Anthony Harris' discussion paper– *On what basis should we decide about health care priorities?* – full paper
- Appendix 3: Other resources
- Appendix 4: Decision-making activity
- Appendix 5: The current decision criteria
- Definitions

1. Introduction

PHARMAC last reviewed its Operating Policies and Procedures, which include our criteria for deciding which pharmaceuticals are available for public subsidy, in 2005. So, in 2012, PHARMAC launched a review of its OPPs with a period of public consultation. The feedback we received indicated that people were interested in having a say about how PHARMAC works, particularly with respect to our decision criteria, what they take into account and how we use them. As a result, we are asking the people with work with (and for) for their views on whether the decision criteria we use for making these important decisions still reflect the things that the public feel are important. Community values are constantly changing and it is essential that we test these criteria in the public domain to check that they are still relevant.

Moreover, the Government has asked PHARMAC to pick up areas of medical funding outside its traditional focus on pharmaceuticals used in the community and cancer medicines used in hospitals. In July of this year, we will implement a nationally consistent list of funded hospital medicines, and we are working towards managing these under a fixed budget, in the same way that we manage community pharmaceuticals. Consultation with DHBs is on-going. PHARMAC has already commenced work on national procurement of some medical devices. During 2015, PHARMAC will commence assessment of new hospital devices moving towards full budget management (within a budget agreed with DHBs and the Minister of Health) at the start of the 2017 financial year.

As a result of these changes to what PHARMAC does it is important that we ensure that how we do these things remains relevant.

This consultation document forms the basis of a conversation we hope will occur around our decision criteria; a conversation that will result in feedback to help us tailor our funding decision-making criteria for better health outcomes. To do this we have engaged a prominent, independent health economist, Professor Anthony Harris from Monash University, Melbourne, to provide an overview of the challenges facing those charged with making health spending decisions. You will find this in Appendix 2.

This document is also designed to provide sufficient background to our decision criteria so that you gain some understanding of their current use and purpose. Some people may feel that they already have a good idea about how PHARMAC's decision criteria are applied; some may feel they have no insight at all. Whatever your experience, we are looking for your comment on how the decision criteria might better help PHARMAC fulfil its statutory objective of securing the "*best health outcomes ... from pharmaceutical treatment ... within the amount of funding provided.*"

2. Context and the review process

2.1. Origins and aims

PHARMAC is currently reviewing its Operating Policies and Procedures (OPPs). These are PHARMAC's framework for how we carry out our statutory role of deciding, on behalf of District Health Boards, which pharmaceuticals and related products are subsidised for use in the community and by public hospitals.¹ They provide guidance to the people and groups with whom we work about what to expect when working with us, and they steer us internally as we consider funding proposals and policy changes. They need to reflect our expanding role in relation to medical devices and hospital medicines.

PHARMAC's decision criteria are part of our Operating Policies and Procedures.

2.2. Process to date

The formal review of our OPPs began with a discussion at the PHARMAC Forum on 20 February 2012. In April 2012, we released a discussion document seeking feedback from the public on what should be included. In response to the submissions we received, in early December 2012, we released notification of:

- The list of topics to be included in the revised OPPs
- Our intention to re-develop the OPPs as a web-based guide
- Our intention to begin a review of the substantive content of the OPP topics (and thus PHARMAC practice), starting with a review of our nine decision criteria

You can find the summary of the feedback provided [here](#).

We decided to begin with a substantive review of PHARMAC's decision criteria because this was a topic of great interest, and we consider that our decision criteria feed into the content of all the other policies and processes we use to achieve our statutory objective.

2.3. Seeking your feedback

This document outlines what our decision criteria are, why we are reviewing them and how you can get involved and have your say.

We have tried to describe the current decision criteria, and how they are used, in sufficient detail to enable you to provide an informed response. Nothing in this document is intended to direct your response, or eliminate anything from discussion. We want to know what you think of the current decision criteria, if they are still fit for purpose and if there are any changes, additions or deletions you think we should make to them. Questions to help your thinking are collated in Appendix 1.

Please pass on this document to anyone you think may be interested.

¹ From July 2013 in respect of public hospitals.

2.4. Submitting your response

Comments can be submitted through our online consultation form or via email, fax or letter by 5pm Friday, 30 August 2013 to:

<http://www.pharmac.health.nz/about/operating-policies-and-procedures/decision-criteria-consultation>

Nikki Shute Email: opp@pharmac.govt.nz

PHARMAC Fax: (04) 460 4995

PO Box 10-254

Wellington 6143

We also invite interested people or groups to meet with PHARMAC staff to present their views in response to this consultation. Please contact Nikki Shute on (04) 916 7513 by Friday, 28 June 2013 if you would like to arrange a time to meet with us. If a range of groups are interested in meeting we may organise larger group meetings.

We will be holding community forums as part of our decision criteria consultation. To find out more about these:

<http://www.pharmac.health.nz/about/operating-policies-and-procedures/decision-criteria-consultation>.

Please contact Nikki Shute via the above details, if you require any further information about any other aspects of this review.

Information requested under the Official Information Act

Feedback we receive is subject to the Official Information Act 1982 (OIA) and we will consider any request to have information withheld in accordance with our obligations under the OIA. Anyone providing feedback, whether on their own account or on behalf of an organisation, and whether in a personal or professional capacity, should be aware that the content of their feedback and their identity may need to be disclosed in response to an OIA request.

We are not able to treat any part of your feedback as confidential unless you specifically request that we do, and then only to the extent permissible under the OIA and other relevant laws and requirements. If you would like us to withhold any commercially sensitive, confidential proprietary, or personal information included in your submission, please clearly state this in your submission and identify the relevant sections of your submission that you would like withheld. PHARMAC will give due consideration to any such request.

2.5. Next steps

After the consultation period closes, we will consider all submissions, release a summary of the submissions received, and provide proposals for any changes to PHARMAC's decision criteria to the PHARMAC Board. If changes to PHARMAC's decision criteria are recommended as a result of this consultation, we will undertake a second round of public consultation on what those changes might be, before going back to PHARMAC's Board for a final decision. We will be able to advise a likely timeframe for implementing any changes following the end of this second round of consultation.

Some people are interested in participating in the review of other areas of our OPPs. As the decision criteria are a central part of our OPPs, any changes that might be made to the decision criteria will need to be taken into account when reviewing other sections of our OPPs. This

means that other areas of our OPPs will be reviewed following the completion of the decision criteria review.

3. Professor Anthony Harris' discussion paper – executive summary (for the full article see Appendix 2)

The opinions and views expressed in the following article (being a summary – the full article is set out in Appendix 2) are those of the author and do not necessarily reflect the opinions or views of PHARMAC.

On what basis should we decide about health care priorities?

What should be the principles that decide treatment priorities and how should we use the idea of need to set them? Perhaps the most obvious principle that might be applied to these decisions is to pay only for those treatments that improve health and, within our limited budget, choose the ones that improve health the most.

This is the key to the approach that has been taken to medicines in New Zealand by PHARMAC, as well as in Australia, and in many countries in Europe including Britain, the Netherlands, Portugal, the Scandinavian countries, as well as in Israel and Mexico. There are variations across countries in how these decisions are made, but in general the aim has been to provide medicines at low cost to all, but to choose which medicines to subsidise by assessing their comparative value. Value has been taken to mean the cost of achieving a gain in years of life and the quality of life.

But this efficiency principle has been challenged by the view that those who are in greater need, such as those who have a life threatening condition like terminal cancer, should get priority over those with a non-life threatening chronic condition like asthma even if the cost is greater and the measurable health gain is less. There seems to be a general consensus that need, as measured by severity of illness, is something that should be considered in addition to the cost and actual gains in quality of life from treatment.

Further some have argued that we should consider not just those in immediate need, but also those who have experienced long term disadvantage or disability prior to treatment. So for example we might also want to give priority to Māori and Pacific Peoples or children with cystic fibrosis both of whom in different ways have experienced long term disadvantage or disability prior to treatment.

In deciding how we should set priorities, and what we mean by 'need' in that process, international and local experience suggests that we might want to consider whether:

- the notion of looking for efficiency in health spending is accepted?
- people believe that the severity of the illness should be a factor (in addition to cost and the effectiveness of treatment) in determining which medicines are covered by PHARMAC?
- people believe that need also expresses people's whole of life experience and not just the current prognosis?

Profile: Professor Anthony Harris

Anthony Harris has held teaching and research positions at the universities of Aberdeen, Western Australia and Murdoch. He is currently teaching at Monash University, Melbourne.

He has been closely involved in the application of health economics to health technology assessment through the use of decision analytic modelling and economic analysis alongside clinical trials. He has published widely in health economics, particularly in the area of health services decision making. His most recent work focussed on the link between health, health care utilisation and labour outcomes.

In 2009, he was awarded an Australian Research Council grant to look at the determinants of negotiated drug prices in Australia.

4. Background

4.1. Introduction to PHARMAC

The Pharmaceutical Management Agency (PHARMAC) is the New Zealand Crown entity that decides, on behalf of District Health Boards (DHBs), which pharmaceuticals and related products are subsidised for use in the community and public hospitals.

Our key obligations are set out in the New Zealand Public Health and Disability Act 2000 (NZPHD), which states that our core objective 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 amount of funding provided

NZPHD section 47(a)

Our key statutory functions are, within the funding provided to us, to:

- a) *Maintain and manage a pharmaceutical schedule that applies consistently throughout New Zealand, including determining eligibility and criteria for the provision of subsidies*
- b) *Manage incidental matters arising out of paragraph (a), including in exceptional circumstances providing for subsidies for the supply of pharmaceuticals not on the pharmaceutical schedule*
- c) *Engage as [we see] fit, but within [our] operational budget, in research to meet our objective*
- d) *Promote the responsible use of pharmaceuticals*
- e) *Any other functions [we are] for the time being given by or under any enactment, or authorised to perform by the Minister by written notice to the board of PHARMAC after consultation with it.*

NZPHD section 48

As a Crown entity, PHARMAC has a commitment to upholding the principles of the Treaty of Waitangi. PHARMAC's Māori Responsiveness Strategy, Te Whaioranga, provides a framework for ensuring that PHARMAC responds to the particular needs of Māori in relation to pharmaceuticals.

We carry out our responsibilities through a wide range of activities, including, among others, the clinical and pharmoeconomic assessment of pharmaceuticals, commercial procurement strategies, negotiations with pharmaceutical suppliers, access and optimal use of medicines strategies, and contributing to advice to the Government on relevant matters.

PHARMAC does not decide which pharmaceuticals are safe for use in New Zealand. This is the role of Medsafe. PHARMAC is responsible for deciding which pharmaceuticals should be subsidised for use in New Zealand.

5. PHARMAC's decision criteria

5.1. The purpose of the decision criteria

In deciding which pharmaceuticals to fund, PHARMAC seeks to balance the needs of patients and communities with its responsibilities to the taxpayer. PHARMAC's decisions need to represent good value for money for the health benefit of all New Zealanders.

PHARMAC uses the decision criteria set out below, to make decisions about proposed amendments to the Pharmaceutical Schedule and decisions outside the Schedule relating to treatments for named patients. Where PHARMAC makes decisions that do not involve amendments to the Schedule or named patients (for example, decisions relating to PHARMAC's access and optimal use activities), it tries to use these criteria, to the extent that they can be applied to those decisions. The decision criteria were developed prior to PHARMAC expanding its role into hospital pharmaceuticals and medical devices.

The nine decision criteria are:

1. The health needs of all eligible people within New Zealand
2. The particular health needs of Māori and Pacific Peoples
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 to PHARMAC, or in PHARMAC's Funding Agreement, or elsewhere
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.

The decision criteria take into account the factors PHARMAC considers are relevant when making pharmaceutical funding decisions in a New Zealand context.

5.2. When and how PHARMAC uses the decision criteria

Pharmaceutical Schedule applications

Each year, PHARMAC receives a large number of applications to fund new pharmaceuticals, or to widen access to pharmaceuticals that are already funded. As PHARMAC must work within a fixed budget, difficult decisions need to be made. This involves assessing a large amount of often complex information to identify those proposals that would provide the best health outcomes.

Initial funding applications and the decision criteria

The most important use of PHARMAC's decision criteria is when we are making funding decisions. Our Therapeutic Group Managers (TGMs) use them when taking a funding proposal to the Pharmacology and Therapeutics Advisory Committee (PTAC) and PHARMAC's Board.

The types of questions that arise are:

- Is there a need for this new pharmaceutical? (DC 1, 2, 3, 8)

- Is this patient group already well-served by existing, already funded pharmaceuticals? (DC 3, 4, 5, 6)

- In terms of health budget spend, is the cost to the community sustainable? (DC 5, 6, 7)

When PTAC and the PHARMAC Board consider a funding application, they also refer to the decision criteria.

In deciding which pharmaceuticals to fund, PHARMAC assesses each proposal against each decision criterion. Having one set of criteria for all decisions, allows PHARMAC to be as consistent and objective as possible. All proposals are prioritised against all other funding options according to these criteria.

In the 'lifetime' of a funding application, there are several occasions when PHARMAC staff and the PHARMAC Board will turn to the decision criteria for guidance. These include:

1. When PHARMAC staff undertake an initial report on an application
2. When the Pharmacology and Therapeutics Advisory Committee (PTAC) (and/or the relevant Subcommittee) makes its recommendation
3. During the prioritisation process
4. When PHARMAC makes its funding decision.

NB The process around considering funding applications is not the subject of this consultation, and is provided to help inform your understanding of the purpose and intent of the decision criteria. Other PHARMAC processes, such as the prioritisation process, will be the subject of a future phase of the OPP review.

1. Initial analysis by PHARMAC

Once a new funding application has been received, the application is referred to someone in PHARMAC's Operations Directorate who will seek more information and work with other PHARMAC staff to collate that information in order for the application to be considered by PTAC (refer below for more information). In some cases, the application also goes to one of the specialist PTAC Subcommittees.

Amongst other factors, PHARMAC staff consider the nature of the disease for which the pharmaceutical has been developed, the treatments already available in New Zealand for that disease, the cost of funding the pharmaceutical under consideration and its cost-effectiveness: all of which are captured by the decision criteria.

2. The Pharmacology and Therapeutics Advisory Committee (PTAC)

PTAC is an expert medical committee that provides objective advice to PHARMAC on health needs and the clinical benefits of particular pharmaceuticals. Committee members are all senior, practising clinicians who help in the process of deciding which pharmaceuticals are to be subsidised by making recommendations to PHARMAC.

When considering an application, PTAC will review and critically appraise the clinical evidence and assess its relevance to the New Zealand health sector setting. It uses the same decision criteria as PHARMAC when evaluating applications. When recommending a particular pharmaceutical be funded, or not, or whether an expanded group of patents should gain access to the pharmaceutical, and deciding on its priority for funding, PTAC indicates which decision criteria it has found to be particularly relevant for the recommendation being made. These recommendations are taken into account when PHARMAC sets its funding priorities. A PTAC recommendation to fund a pharmaceutical does not necessarily mean that it will be funded as it may be given a low priority in the next step in the process.

3. The Prioritisation Process

When information on an application is available (including PTAC's recommendation and cost-effectiveness, as necessary), it is compiled and considered by PHARMAC. At this time, all new

applications are prioritised against all other assessed funding options using the decision criteria to help determine the relative ranking of each application. The aim is to identify potential changes to the Schedule that would provide the best health gains.

PHARMAC regularly reviews all applications, which can result in a reordering of the relative ranking of applications: some pharmaceuticals may shift down the list, others may move up. The prioritisation discussion involves people from across PHARMAC – management, Therapeutic Group Managers, Medical Directors, health economists and analysts.

4. *The PHARMAC Board*

If PHARMAC considers an application should proceed, PHARMAC staff will often negotiate with the supplier to reach a provisional agreement. If PHARMAC considers it appropriate to do so, PHARMAC will then consult with relevant sections of the health sector on the proposal, taking this feedback into account before a decision is made. Final decisions are usually made by the PHARMAC Board or by the Chief Executive acting under delegated authority of the Board. As with the prioritisation process, the PHARMAC Board will review the full list of pharmaceuticals recommended for funding each time it meets.

Named Patient Pharmaceutical Assessment (NPPA)

In addition to managing the Schedule, PHARMAC is also legislatively required to provide subsidies in exceptional circumstances for pharmaceuticals not listed on the Schedule. PHARMAC does this through its NPPA policy. The purpose of the NPPA policy is to ensure an individual's clinical circumstances can be considered, if those circumstances have not been considered through the Schedule process (either because the individual's clinical circumstances are relatively rare, or because they require a decision more quickly than the time it takes to consider the pharmaceutical through the normal Schedule process).

Decisions on NPPA applications are usually made by PHARMAC staff under the delegated authority of the PHARMAC Board. In making a NPPA decision, PHARMAC staff first consider whether the applicant meets the pre-requisite criteria set out in the NPPA policy; only then is an application assessed against the nine decision criteria. PHARMAC will use the decision criteria to assess both the individual clinical circumstances of each NPPA application and the implications of each NPPA funding decision on PHARMAC's ability to meet its objective for the New Zealand population as a whole.

5.3. Medical devices

PHARMAC will, from July 2015, take on responsibility for the prioritisation of medical devices used in DHB hospitals. This will extend to full management of a fixed budget for medical device expenditure on behalf of DHBs from July 2017. Therefore, a key area for feedback that is currently being sought is how the current decision criteria might apply to medical devices.

In a variety of obvious and less obvious ways, devices are different to pharmaceuticals. For example, there is much less data available, as a result of fewer blinded randomised controlled trials that can be sensibly undertaken, to clearly determine how effective particular devices are.

Try your hand at PHARMAC-style decision making

Appendix 4 is an activity you might like to try, to stimulate your thinking about what your own set of pharmaceutical-funding decision criteria might include.

The example is not a sophisticated one, and in that respect, it does not reflect the true nature of the decision making PHARMAC faces. However it might help you start thinking about what is important to you when thinking about which pharmaceuticals to fund, or not to fund.

Other considerations are particularly relevant for medical devices, such as whether significant and/or on-going technical support, or user training for patients or clinicians, is required. It may therefore be much more difficult to attribute direct health gains to a particular device. Further considerations can include whether the device would require PHARMAC to purchase a number of other 'accessories' and the expected lifespan of the device, i.e. how quickly would technology advances make this device obsolete? If a medical device is a capital investment, PHARMAC may also need to consider how it should take into account the on-going maintenance and service costs.

PHARMAC is interested to hear whether people think there should be a different set of decision criteria for devices and, if so, what they might include and how they might be measured.

5.4. Other considerations

Professor Anthony Harris' article, in Appendix 2 of this consultation document, raises a number of pertinent questions about PHARMAC's decision criteria, particularly around how fairness and community values form part of the decision-making process. These are issues that have always been important to communities and to the organisations that represent them, including PHARMAC. How should fairness and community values be taken into account when making decisions around securing "for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided"?

PHARMAC is also interested to hear your feedback on what other considerations you think PHARMAC should be taking into account in its OPP decision criteria. For example, should we take into account the ability of parents of sick children to return to work (or not take time off at all)? Should we assess the future earning potential of children? If we do that, how would we then consider the on-going contribution made by people no longer in paid employment and those whose earning potential is low because they are already sick, or have been sick for many years? How should PHARMAC take into account the variety of different community values of different groups of people, such as beliefs about contraception, or providing treatment for conditions that some might label 'preventable', and those that are genetically-based? What measures could be put in place to quantify these values? And, what about the broader, non-health, benefits to society of some treatments, such as moving people off sickness benefits and back into paid employment?

Appendix 1: Guiding questions

Keeping in mind PHARMAC's statutory objective – 'to secure for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided' – tell us what you think about the following questions and anything else you would like to tell us:

1. What are your views on the value of the current nine decision criteria?
2. What have been your experiences with our current decision criteria?
3. To what extent should the criteria give PHARMAC the flexibility to make decisions on a case-by-case basis, and to exercise judgement?
4. Is there anything about the nine existing criteria that make them inappropriate to be applied to medical devices? Why?
5. What other criteria might be needed when considering the priority of a medical device?
6. What advantages or disadvantages would there be in all PHARMAC's decisions, for pharmaceutical and devices, being made using the same set of criteria?
7. How specific should the criteria be? How general should they be?
8. What other criteria should/could PHARMAC consider?
9. Of the current criteria, which remain appropriate to retain? Why? Which ones are no longer appropriate? Why?
10. If you were to have a clean slate, around what criteria would you base decisions for funding pharmaceuticals within a fixed budget?
11. How do the criteria currently reflect fairness or community values?
12. What additional criteria would you suggest to reflect fairness or community values and how could these be measured?
13. What additional information or detail do you think should be included in the decision criteria section of the OPPs?

Appendix 2: Professor Anthony Harris' discussion paper – On what basis should we decide about health care priorities? – full article

Universal health care

The New Zealand health system has been built on the principle of universality. It recognises that sickness and accidents can happen to any of us, and that everyone should have access to the same standard of care when they need it, irrespective of their ability to pay.

This principle of universality does not mean that the public should pay for every test, treatment or medicine that improves health, no matter the price or how effective it is. Health care has become so sophisticated and expensive that no country (not even the richest) can afford all the potentially beneficial medical procedures that are now available, for all the people who might possibly benefit from them. We need to set priorities on what we will pay for, and for whom.

A key question for the health system is:

What should be the principles that decide treatment priorities?

- Should we give priority in health services to those who can benefit most or those who have the most severe illness?
- Should we give priority in health services to those who are disadvantaged not just in health but in life circumstances more broadly?
- Should we give some priority to health services that prolong life for the elderly, or to those that improve quality of life in the young who have their life ahead of them?
- Should we consider the cost of treatment in setting priorities?
- Are there important principles not just about priorities themselves but also about the way in which decisions are made?

Some might deny that we need to make these choices and that we can and should provide care to all who need it. They might argue that we can always afford more if we reduce waste in the system or become more productive. It is true that we can afford more by stopping medical procedures that we know don't work or unnecessary tests that give no useful information. We can also become more productive through innovation, but there are limits to these kinds of efficiency savings.

Many countries, including New Zealand, have now accepted that explicit priority setting - prioritising patients on waiting lists for surgery or excluding certain medicines - is necessary if access to an acceptable level of care is to be guaranteed to all. So while the principle of universality means that everyone who needs care should have access to it, even if they can't afford to pay for, it does not tell us how to decide who is most in need. There are always questions about whether we should use our resources on a new expensive treatment that offers some benefits to one group of patients, rather than provide something to another group who are perhaps more needy, more numerous, or have a greater potential to benefit.

The purpose of PHARMAC's consultation exercise is to get the views of the New Zealand people on the principles upon which we can make these kinds of decisions.

The efficiency principle

Perhaps the most obvious principle that might be applied to these decisions is to pay only for those treatments that improve health and, within our limited budget, choose the ones that improve health the most.

This is the key to the approach that has been taken to medicines in New Zealand by PHARMAC, as well as in Australia, and in many countries in Europe including Britain, the Netherlands, Portugal, the Scandinavian countries, as well as in Israel and Mexico. There are variations across countries in how these decisions are made, but in general the aim has been to satisfy the first principle of universality by providing medicines at low cost to all, but to choose which medicines to subsidise by assessing their comparative value. This raises the question of what we mean by the “value of medicines”, or how we measure and value their outcomes.

All of these countries have tended to value health outcomes according to the years of additional life gained for each individual treated and the quality of life in those additional years. The value has usually been calculated relative to the cost of achieving those outcomes. This is often called the ‘efficiency approach’ where we compare the cost and health outcomes of treatment with the cost and health outcomes of the next best alternative. The principle is that we would only fund a medicine if there are gains in quality of life for patients that come at an acceptable additional cost compared to current treatments.

The efficiency approach

A common approach to measurement of health is to combine (multiply) any additional years of life from treatment with the quality of life during those years. The result is a Quality Adjusted Life Year (QALY) with a scale from zero (dead) to one (perfect health). The efficiency approach to priority setting in health service reimbursement is then to maximise health per dollar (the sum of quality adjusted life years gained (QALYs) per \$ spent). So for example if we as a society are willing to pay no more than \$50,000 per QALY, and we could not get more value elsewhere in the health system, then we should only pay for new medicines that cost up to \$50,000 per QALY. The reason is that with a fixed budget for medicines (as in New Zealand) we need to make sure that new drugs do not displace existing ones that produce at least as many QALYs for less money. Efficiency then means that we would maximise the total healthy years in the population for the amount of money we are prepared to spend on health care.

Using the efficiency principle raises two key issues:

- Does totalling the years of survival across patients even after adjusting for the quality of life in those years reflect everything we want to include in the ‘social value’ of the gains from treatment? Are those who have more severe conditions, such as those who face imminent death without treatment, more deserving of that treatment even if treatment is less effective and more costly?; and
- Should we place a higher social value on some groups of patients and, if so, which ones? How should we treat people who because of social or personal circumstances have not had a chance to live a full and fulfilling life?

Let us consider these two issues in more detail.

Are QALYs adequate?

Even if we accept the principles of universality and efficiency, we might feel that some patient groups, like people with cancer should get more funding than some other groups, even if the gain in QALYs is less, because in some other sense they have a greater need. A common view for example is that those who have a life threatening condition, like terminal cancer, should get more funding than those with a non-life threatening, but long-term debilitating, chronic condition like asthma. In other words we might want to fund some treatments, even if they appear inefficient in that they offer fewer QALYs or perhaps even very little survival gain to patients and cost a great deal, because we feel that these patients have greater or more urgent health needs.

A pressing question for bodies like PHARMAC is:

Should high 'health needs' provide additional weight to decisions to fund some treatments, even where treatment is very costly and perhaps not very effective in improving quality of life or survival?

There is a common feeling that people facing the worst prospects – perhaps only a short time to live - need to be 'rescued' first. In effect this means giving priority to those with the poorest QALY prospects without treatment. An obvious problem with this approach is that severity of illness (or proximity to death) then establishes priority for health care expenditure for a group of patients, irrespective of their capacity to benefit from treatment.¹ The costs of treating patients close to death are often very high, but benefits are often very low because deaths cannot be averted despite all efforts. Giving more funding to patients facing imminent death has a high opportunity cost compared with health gains that could be made from funding treatment for patients with less severe conditions particularly if there are very effective low cost treatments available. We therefore might want to limit the extent of this priority to ensure that there is greater benefit to patients from treatment.

Countries that use the efficiency criterion in deciding what medicines to pay for vary in their approach to the issue of health need. The most common approach is to adjust the threshold for an acceptable cost per QALY based on severity of illness. This means that rather than saying a medicine must have a cost per QALY gained of less than \$50,000 to be made available on the list of drugs that are paid for by government; they might accept a drug for an otherwise untreatable cancer that had a cost of \$80,000 per QALY gained. This is the approach taken formally by NICE in England and Wales and more informally by others such as the PBAC in Australia. In addition many jurisdictions around the world consider the value of pharmaceuticals prior to public subsidy (including New Zealand, Sweden, Scotland, England and Wales, the Canadian Provinces, France, Korea, Turkey, Italy, Israel, Estonia, Portugal, Slovak Republic, Czech Republic, Denmark, Estonia, Norway, Poland, Finland, Ireland). In such jurisdictions a range of health-related outcomes may be factored into decisions about which drugs to subsidise, including severity of illness, proximity to death, availability of alternative therapies and extent of the quality life gain. For example in Sweden the Pharmaceutical Benefits Board (LFN) makes reference to the "human value" principle, i.e. it cannot discriminate against people because of their sex, race, age and so on, when considering a medicine; and the "need and solidarity" principle where drugs that treat those with the greatest health needs take precedence. However in this case it is not precisely clear what is meant by "health need". For example, Sweden, like Australia, has decided to pay for drugs that are used for severe conditions in small numbers of patients in spite of high costs per QALY gained (typically these are cancer drugs). This means that other groups, who have less severe illnesses, may have to pay for their own treatment or miss out altogether. For example Sweden has been unsympathetic to drugs for the treatment of

milder stomach acid symptoms (heartburn for example) even though they have low cost per gain in quality of life.² Another approach, with a similar underlying principle, is in Canada where cancer treatments are considered separately, implicitly allowing for a higher threshold cost per QALY.

There seems to be a general consensus that severity of illness (as prognosis or the potential gains from treatment) is something that should be considered in addition to the actual gains in quality of life from treatment.

As far as I am aware no country has yet tried to include severity of illness (or other notions of equity or justice) into their pharmaceutical decision-making processes, in a comprehensive, systematic way. Some countries have begun to consider more concrete, explicit measures of severity in their decision making. In New Zealand PHARMAC has been considering the use of a range of health status indicators to better capture a notion of 'need as severity of illness' to inform its funding decisions. This could be summarised as:

The gap between the life expectancy (adjusted for the quality of that life) with current standard care for a person of a given age and gender with a particular condition, and the life expectancy of an average, healthy person of the same age and gender.

Lifetime and age

An alternative view is that we should consider not just "need as severity" - measuring the extent of their future health loss from the illness without treatment - but perhaps give some priority to those who have experienced long term disadvantage or disability prior to treatment (Māori and Pacific Peoples might be one example) or who because of the age of onset of the disease will not have the chance for a full and fulfilling life.³ Ways to do this might include giving priority to younger people (and perhaps those who have had previous severe illness or other disadvantages) on the grounds that they deserve the opportunity to have a life – an opportunity that most older people have already had.

There is some evidence that people in other countries are prepared to consider this view of need. For example in a study in the Netherlands⁴ find more support for life time experience of health as a consideration. There is no clear consensus across published studies where they variously show public support for giving some priority to those with a poor prognosis, those who are younger, and those who will gain most from treatment.

Some countries vary the level of subsidy according to patients' income and age; others vary the subsidy according to patient and disease characteristic, even where severity of illness is not a criterion in broad subsidy decisions. Ireland, Denmark, Spain, Finland, and Turkey have lower levels of patient payment for chronic long-term conditions – compared with acute conditions – while Korea, Portugal, and Denmark consider the severity of the disease or the essential nature of the therapy. These lower patient payments in a regulated pricing system imply a higher social willingness to pay for patients with certain characteristics - typically those perceived as having a greater 'level of perceived need'.

This consultation exercise is about your views on what should determine priorities in the funding of medicines in New Zealand.

It is inevitable that in any resource allocation mechanism, such as public medicines reimbursement decisions, priorities are made about groups of patients. Some patients will gain more QALYs than others. It may not be enough to continue with the tacit belief that these QALYs are of equal value no matter who gets them; but if people think it is critical to take account of who will get the health gains then PHARMAC can make better decisions if it includes that information.

It is not obvious however that considering just the QALYs gained per dollar along with the severity of illness (or proximity to death) is acceptable. One issue is that it would establish priority for health care expenditure for a group of patients who might have a limited capacity to benefit from treatment.¹ We might want to limit the extent of this priority to ensure that there is a substantial benefit to patients from treatment. For example, the Citizens Council of NICE, a body designed to capture the informed views of the public in England and Wales, felt by a majority of 24 to two that severity of disease should be considered by NICE independently of the calculation of health gains as measured by QALYs gained.⁵ So in the case of NICE in England and Wales the decision in 2009 to give greater weight to QALYs where the patient's life expectancy is less than 24 months only applies where the treatment would provide at least three months of benefit.

A problem remains if we do want to give some priority to those who have severe illnesses - how much extra weight to give the QALYs of a person with a life threatening illness like cancer? The cost of treating patients close to death is often very high, but benefits are often very low because deaths cannot be averted despite all efforts. Giving more funding to patients at the end of life has a high opportunity cost in terms of health gains that could be made from funding treatment for patients with less severe conditions that are not immediately life threatening but for which there are very effective low cost treatments available. This dilemma has become more apparent in the last decade with the increase in the number of biologic medicines that target groups of patients who will potentially benefit more in terms of survival and quality of life, but with significantly higher costs per patient treated. The issue of targeted personalised therapies more generally may require us to re-think what we value in the health system, as pharmaceutical companies focus on lower volume niche products. These medicines have the potential to be more effective and safer for smaller groups of patients, but the profitability to the companies may depend on high prices, and the consequence may be uncertain cost effectiveness and an increased risk of high costs to the health system. For these reasons it is important that decision makers in the health system have clearer set of criteria on which to base funding decisions.

What should be the principles for spending on medicines?

Most people recognise the need to get value for money from health spending. We feel at least some inclination to help those worse off than those better off. Length of life matters. Quality of life matters. Life itself, even if it is short or of reduced quality, has value, especially to a person who desires it. The purpose of this consultation is then to consider first if these kinds of principles or others reflect the views of people in New Zealand, and more than that, if a set of principles can be formulated in a way that will be useful for PHARMAC in making decisions on which medicines to include in the national formulary.

Some broad questions for this consultation then might be:

- Is the principle of universality accepted?
- Is the criterion of efficiency in health spending accepted?
- Do people believe that the severity of the illness should be a factor in addition to cost and the effectiveness of treatment in determining which medicines are covered by PHARMAC?

- If they do, then should severity be measured by
 - the prognosis of their illness?; or
 - should we rather measure severity by the whole of their life experience and not just the current prognosis? Do we think, for example, that a young person who has had a debilitating condition since birth is more “in need” or deserving than an older person with a life threatening illness who has otherwise had a healthy life?
- Do we think that non health disadvantages should influence our health funding decisions? Should for example poverty or general social disadvantage be a factor in determining health priorities in addition to the broad universality offered by subsidised care in New Zealand?
- What other principles should influence decisions? How much, if at all, does it matter how decisions are made (in public or closed door negotiations) or do we care more about the results of the actual funding decisions?

References

¹ Culyer AJ, Wagstaff A. Equity and Equality in Health and Health Care. *J Health Econ.* 1993;12(4):431-457.

² Docteur E, Paris VR, Moise P. Pharmaceutical pricing policies in a global market. Paris: Organisation for Economic Co-operation and Development (OECD), 2008.
<http://www.oecd.org/health/pharmaceuticalpricingpoliciesinaglobalmarket.htm>

³ Williams A. Intergenerational equity: an exploration of the 'fair innings' argument. *Health Econ.* 1997;6(2):117-132.

⁴ Stolk EA, Pickee SJ, Ament AH, Busschbach JJ. Equity in health care prioritisation: an empirical inquiry into social value. *Health Policy.* 2005;74(3):343-355.

⁵ NICE Citizens Council. Departing from the threshold. Report on Council meeting, November 27-29, 2008. London: National Institute for Health and Clinical Excellence (NICE), 2008.
<http://www.nice.org.uk/newsroom/features/CitizensCouncilReport.jsp>,
<http://www.nice.org.uk/media/231/CB/NICECitizensCouncilDepartingThresholdFinal.pdf>

Appendix 3: Other resources

The resources set out below provide further information that is relevant to the subject matter of this review, and which may help you in formulating your feedback. The opinions and views expressed in these resources are those of the respective authors.

McCormack P, Quigley J, Paul Hansen P. Report to Minister of Health, Hon Tony Ryall: Review of Access to High-Cost, Highly Specialised Medicines in New Zealand. 31 March 2010
http://img.scoop.co.nz/media/pdfs/1005/Review_of_Access_to_High_Cost_Highly_Specialised_Medicines_31_April_2010.pdf

National Institute for Health and Clinical Excellence. Social value judgements: principles for the development of NICE guidance. Second edition, 2008.
<http://www.nice.org.uk/aboutnice/howwework/socialvaluejudgements/socialvaluejudgements.jsp>

NICE Citizens Council. Departing from the threshold. Report on Council meeting, November 27-29, 2008. London: National Institute for Health and Clinical Excellence (NICE), 2008.
<http://www.nice.org.uk/newsroom/features/CitizensCouncilReport.jsp>,
<http://www.nice.org.uk/media/231/CB/NICECitizensCouncilDepartingThresholdFinal.pdf>

Rhodes R, Battin MP, Silvers A (eds). Medicine and social justice: essays on the distribution of health care. Oxford University Press, 2002.
http://books.google.co.nz/books?id=YsQlbtX2GXsC&printsec=frontcover&source=gbs_ge_summary_r&cad=0#v=onepage&q&f=false. (includes: Hope T, Reynolds J, Griffiths S. Rationing decisions: Integrating cost-effectiveness and other values, as Chapter 11 pp 144-155).

Hansen P. A theoretical review of PHARMAC's over-arching approach to deciding which pharmaceuticals to fund, including high cost ones. 2006.
<http://www.pharmac.govt.nz/2006/12/15/HCM.pdf> pp 67-101 (of 191)

New Zealand Organisation for Rare Disorders. New Zealand Rare Disease Day press release, 28 February 2013. Calls for an orphan drugs access policy to overcome Pharmac's systems failure with specialised medicines.
http://www.nzord.org.nz/news_and_issues/releases_and_submissions/2013/new_zealand_rare_disease_day_press_release_28_february_2013

Access to Medicines Coalition press release, 3 August 2006. Ethical principles must come first in medicines strategy.
http://www.atmcoalition.org.nz/news/ethical_principles_must_come_first_in_medicines_strategy

Rasiah D, Edwards RR, Crampton P. Funding community medicines by exception: a descriptive epidemiological study from New Zealand. *New Zealand Medical Journal* 2012;125(1350):21-9.
<http://journal.nzma.org.nz/journal/abstract.php?id=5071>

Appendix 4: Decision-making activity

Making decisions about which medicine to fund is never easy. PHARMAC gathers a lot of information to ensure that the use of decision criteria in the decision-making process is consistent between pharmaceuticals for different therapeutic groups. The following is a simplified activity that you might like to consider while thinking about the decision criteria you would use to make a funding decision between two pharmaceuticals.

You have funding applications for two pharmaceuticals. The cost for each is the same, but you can only fund one from within your budget.

Pharmaceutical 1:

The first pharmaceutical treats a serious condition that affects a small number of people, mainly those aged over 50 years. The condition worsens as they grow older. By funding it you will over time, moderately improve their and, in later years of the condition, their carers' quality of life, and by preventing some early deaths will extend their lives by an average of 5 per cent.

Pharmaceutical 2:

The other pharmaceutical is targeted at a condition that mostly affects people from the age of 30. Although it does not shorten lives, the condition compromises people's quality of life, preventing them from fully taking part in the community. While the number of studies undertaken has been small, they show that, most people taking the pharmaceutical are quickly able to resume their normal life, particularly in terms of employment.

- Which one would you fund and **WHY**? What criteria are important to you?
- How do you balance the need for patients to have access to healthcare against PHARMAC's statutory obligation to do this within the funding available?

Appendix 5: The current decision criteria

1. The health needs of all eligible* people within New Zealand
2. The particular health needs of Māori and Pacific peoples
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 to PHARMAC, or in PHARMAC's Funding Agreement, or elsewhere
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.

* As defined by the Government's then current rules of eligibility

Definitions

An indication

In medicine, an indication is the reason behind why a clinician undertakes a particular treatment, or diagnostic test. The indication is the clue, i.e. the specific symptom or the doctor's understanding of the cause of the disease or the nature of the disease itself, which points to a treatment or diagnostic test. For example, a clinician may prescribe a Tumour Necrosis Factor (TNF) Inhibitor (the pharmaceutical) for the indication of severe rheumatoid arthritis.

Medsafe

Medsafe is the New Zealand Medicines and Medical Devices Safety Authority. It is a business unit of the Ministry of Health and is the authority responsible for the regulation, safety and efficacy of therapeutic products in New Zealand, i.e. medicines and medical devices.

NPPA

The Named Patient Pharmaceutical Assessment (NPPA) provides a mechanism for individual patients to apply for funding for medicines not listed in the Pharmaceutical Schedule (either at all or for their clinical circumstances). There are three pathways to NPPA funding:

1. Unusual Clinical Circumstances;
2. Urgent Assessment; and
3. Hospital Pharmaceuticals in the Community.

Each has its own prerequisite requirements. Applications that meet the relevant prerequisites, as described in the NPPA Policy, are considered against PHARMAC's decision criteria for funding.

Pharmaceuticals

For the purposes of this document, 'pharmaceutical(s)' refer to both medicines, vaccines and medical devices, unless explicitly stated otherwise.

PTAC

The Pharmacology and Therapeutics Advisory Committee (PTAC) is an expert medical committee that provides objective advice to PHARMAC on health needs and the clinical benefits of particular pharmaceuticals for use in the community and/or DHB Hospitals. Committee members are all senior, practising clinicians who, together, help in the process of deciding which community pharmaceuticals are to be subsidised from public monies by making recommendations to PHARMAC.

The Schedule

When PHARMAC refers to the Schedule we mean the Pharmaceutical Schedule, which lists:

1. The pharmaceuticals available in the community and hospital cancer medicines subsidised by the Government with funding from the Combined Pharmaceutical Budget; and
2. Some pharmaceuticals purchased by District Health Boards for use in their hospitals, and includes those Hospital Pharmaceuticals for which national prices have been negotiated by PHARMAC.
3. From 1 July 2013, the national list of medicines able to be prescribed in DHB hospitals.

Making an **amendment to the Schedule** refers to decisions that PHARMAC makes that lead to a change to the Schedule, either an addition to or deletion of a pharmaceutical from the Schedule or a change to eligibility criteria (the types of patients or diseases or severity).

Pharmaceutical Management Agency

Level 9, 40 Mercer Street, PO Box 10-254, Wellington 6143, New Zealand

Phone: 64 4 460 4990 - Fax: 64 4 460 4995 - www.pharmac.govt.nz

Freephone Information line (9am-5pm weekdays) 0800 66 00 50