

PHARMAC

Pharmaceutical Management Agency

HOW SHOULD HIGH COST MEDICINES BE FUNDED?

PAPER FOR PUBLIC CONSULTATION
DECEMBER 2006

Summary

How and on what basis should PHARMAC make decisions on funding “high cost” medicines? In particular, do high cost medicines require a different approach to their funding?

High cost medicines are those medicines that, unless subsidised by the Government, would be unaffordable for most New Zealanders. Government funding may also be unaffordable if other medicines or health interventions are a better investment. However it is not just the total cost that is important: an equally important consideration is whether it is appropriate to fund high cost medicines for a few people (assuming funding is available) at the expense of lower cost medicines that benefit many more people.

PHARMAC’s work to date, including independent input from a range of experts, suggests that high cost medicines should be treated no differently to other medicines. In other words, the higher cost of some medicines is not justification in itself to adopt a different funding approach.

To further test this view, PHARMAC is now seeking public submissions by Monday 5 March 2007. PHARMAC will report on the submissions and any further steps. This is work in progress: the dialogue about social choices between decision-makers and those affected by decisions needs to continue. PHARMAC acknowledges the Government’s work on developing a medicines strategy – where the strategy work may touch on issues of relevance to this paper, and vice versa.

Description of the problem

Over recent years, an increasing number of medicines have carried a very high cost – some to the point (unless Government-funded) of being unaffordable for most New Zealanders.¹ For some medicines, even Government funding (given limitations and other priorities) may not be possible. This is not an issue unique to New Zealand: it is a challenge confronting all medicines funding systems worldwide (Appendix One illustrates one approach²).

PHARMAC is a public agency that currently funds a number of high cost medicines, through a variety of different mechanisms, including Special Authority, specialist panel management and named prescribers. There is no formal dollar value at which a pharmaceutical is termed “high cost”, as over time what constitutes high cost has and will change. Funding of a medicine 5 years ago at \$20,000

¹ There are other reasons why a medicine may appear to be ‘high cost’: (1) a high cost in aggregate for the budget (but possibly the result of a large number of users); (2) limited health gain compared with existing medicines (such that the cost for the incremental benefit seems high); (3) a wide range of cost-effectiveness estimates. This paper focuses on the classic definition: where the cost of the medicine as such is high.

² For example, the United Kingdom’s National Institute for Clinical Excellence (NICE) document on social value judgments contains 13 principles to use when developing NICE’s guidance (National Institute for Clinical Excellence. Social value judgements: principles for the development of NICE guidance. December 2005. URL: <http://www.nice.org.uk/page.aspx?o=283494>). The extensive literature internationally reflects the ongoing debate in this area – see for example Mortimer D. The value of thinly spread QALYs. *Pharmacoeconomics*. 2006;24(9):845-53.

for each person over a year was very high cost, while now it is much more in the order of \$20,000 to \$100,000. In future, "high cost" could be much more.

High cost medicines are often used to treat rare medical conditions, or conditions for which no effective alternative treatments are available, and some for conditions affecting only a very small number of people. While a small number of patients can, in some cases, make Government-funding of high cost medicines affordable (assuming such investments are good value for money), on other occasions the cost can be tens of millions of dollars each year. Novel treatments, for cancers in particular, are likely to increase the cost of such medicines in the future. The funding challenge will therefore remain and, if anything, become greater.

However, it is not just the total cost that is important. An equally important consideration is whether it is appropriate to fund high cost medicines for a few people (assuming funding is available) at the expense of lower cost medicines that benefit many more people. The difficulty in making these decisions can be highlighted by the often very public debate surrounding individual, high profile cases. To what extent should a public agency consider the needs of the many compared with the needs of the few? And how and on what basis should it make such decisions? These are by no means easy questions, but ones that PHARMAC has no choice but to address.

PHARMAC has been reviewing how it goes about assessing and funding high cost medicines. The cost of these medicines can be so high that the health gain, even if relatively large, is swamped by the cost of the medicine and so becomes, by some definitions, "poor value for money". Positive decisions, therefore, rely on other decision factors and judgements.

PHARMAC's early work in this area also identified the increasing range of new medicines falling into the 'high cost' category. Advances in technology suggest there will be increasing numbers of genetically targeted and other new medicines developed to treat small numbers of patients at very high cost. Were such medicines funded, an increasingly significant proportion of the pharmaceutical budget would be devoted to a relatively small number of patients (without necessarily maximising the 'value' from available funding).

Having your say

Having reflected on this paper and the expert reports (described later), PHARMAC would welcome public submissions by Monday 5 March 2007. PHARMAC will then consider submissions and report on them and further steps, if any, in relation to this work.

The following questions may provide a good focus for submissions:

1. How should PHARMAC approach the trade-off between funding the treatment of very small numbers of patients with very expensive medicines (for very rare conditions) against the treatment of large numbers of patients with less expensive medicines (for more common conditions)?
2. Do you agree with PHARMAC's preliminary conclusion (see the end of this consultation paper) that there are no persuasive arguments for treating the funding of 'high cost medicines' differently to other medicines? If you disagree, then:
 - What information do you think should have been presented by the expert reports and considered by PHARMAC?
 - Which additional particular considerations and/or criteria, specific to assessing and funding high cost medicines, should PHARMAC take into account? or explicitly not take into account?
 - What evidence supports your views?

Please send your submission by e mail to highcostmedicines@pharmac.govt.nz, or mail to:

High Cost Medicines Review
PHARMAC
PO Box 10-254
Wellington

PHARMAC is open to hearing about wider issues about PHARMAC's operations (a number were raised in independent reports commissioned as part of PHARMAC's work), to the extent that submitters feel these are relevant to considering how to fund high cost medicines. For the avoidance of doubt, this is not a review of PHARMAC's overall operations, although PHARMAC acknowledges that a range of issues may be considered relevant.

Background

New Zealand has a national health system where people are funded irrespective of their ability to pay. Pharmaceutical subsidies have been part of this universal scheme – in existence since 1938 – from the outset.

PHARMAC's principal duty is to secure the best health outcomes achievable from pharmaceutical treatment for the population of New Zealand, within the amount of funding it is allocated to manage (see the New Zealand Public Health and Disability Act 2000). The vehicle for this funding is the New Zealand *Pharmaceutical Schedule*, which identifies all community medicines that are funded in New Zealand and the criteria under which they are funded.

In deciding which medicines to fund, on what terms and to who they should be made available, PHARMAC makes a decision on behalf of New Zealand. That decision involves weighing up decision criteria, including assessing the benefits and costs of particular medicines. Nine Decision Criteria are considered.³

PHARMAC's process for making decisions is based on a strong core of clinical advice (sought from its clinical advisory committee, PTAC) and sophisticated methods of critically appraising evidence and assessing pharmaceutical cost-effectiveness.⁴ Decisions cannot, however, be made on the basis of a technical assessment alone: they always involve explicit and implicit value judgements. This inevitably includes particular judgements about the needs, rights and privileges of the many against the needs, rights and privileges of the few.

As such, PHARMAC has always had to grapple with the issue of whether to fund medicines that are more expensive than others. The high cost dilemma is no different conceptually to what it has always been, but the magnitude of what is "high cost" has changed and looks to be changing further.

External input

Process

In addition to PHARMAC's own thinking, reports were commissioned from national and international experts. These reports are now available to help inform public submissions.

³ PHARMAC's Operating Policies and Procedures, the document that sets out PHARMAC's role and objectives, and which contains the details of the nine Decision Criteria, is available at www.pharmac.govt.nz.

⁴ Cost effectiveness is determined by economic analysis, which at PHARMAC usually involves a cost-utility analysis (CUA). CUA is a technique widely used internationally, designed to provide information on the relative value for money of a pharmaceutical – that is, whether the health gains associated with a treatment are greater than the health gains from alternative options that could have been funded with that money. A CUA provides information on the additional quantity and quality of life gained, and resources freed up in the pharmaceuticals budget and elsewhere in the health sector, to the additional cost of the medicine. The methods used when undertaking CUA are outlined in PHARMAC's Prescription for Pharmacoeconomic Analysis (PFFA), available at http://www.pharmac.govt.nz/pharmo_economic.asp. Cost effectiveness is one of PHARMAC's nine decision criteria.

There were two central reports to the review – by Professor Raanan Gillon, emeritus professor of medical ethics at Imperial College (London) and Dr. Paul Hansen, Associate Professor at the Department of Economics, University of Otago. At the core of the reports was the question: should high cost medicines be funded differently from other medicines competing for the same public funding and, if so, for what reasons?

The two lead reports were reviewed by nine external (including two international) peer reviewers representing a range of perspectives and expertise (see Appendix Two for details). The two lead authors were then offered the opportunity to revise their papers in light of reviewers' comments. All of these reports are released with this paper (Appendix Three).

Key findings

There were a number of common themes in the lead reports, as well as the subsequent commentaries:

- ultimately, all decisions are value judgements and entail many considerations beyond technical data and analytical assessments;
- there is no universally accepted mechanism for funding high cost medicines and decisions will depend on the set of values used. There is no single, universally accepted ethical theory on which PHARMAC should base its decisions or which would dictate a particular approach to 'high cost medicines' funding decisions;
- there was consensus that it is appropriate and justifiable to make funding decisions in the context of a finite budget, and an acknowledgement that some difficult moral choices were unavoidable;
- support for a framework (that is, decision criteria) for making such decisions;
- there is a very broad range of competing and sometimes contradictory values, ethical norms and theories of social and distributive justice. All enjoy considerable social standing and acceptance and could legitimately be used to inform the social choices to be made in medicines funding decisions;
- because resources are limited, not all competing claims can or will be met. No matter how carefully and robustly resource allocation decisions are made, it is highly likely that there will be some dissatisfaction with any decision. This is because any decision will generally involve the over-riding of claims with some moral justification in favour of other claims, with other moral justifications, but judged stronger in the particular circumstances;
- it is important that the value judgments that inform social choices are made as explicitly and transparently as possible; and it is particularly important to be aware of possible implicit or hidden value judgements embodied in particular decisions; and
- overall, there is no justification, whether in ethics or economics, for assessing 'high cost medicines' any differently from other pharmaceuticals.

Ancillary issues raised in reports

To restate, PHARMAC's main question at this time is whether the funding of high cost medicines – assuming PHARMAC's processes and decision-making criteria are as they are – need to be treated in a different way to other medicines. On this particular point, the reviewers generally agreed there was no reason for treating these differently.

The authors felt there were a number of other matters related to PHARMAC's activities that were interesting to consider. There were also suggestions about clarifying the value judgements embodied in PHARMAC's decision making processes (including patient "need"), increasing public

understanding around PHARMAC’s decisions; and getting better dialogue about social values and choices.

A summary of our understanding of many of the ancillary issues raised in the external reports is set out below, along with a brief PHARMAC comment. The commentary is preliminary in nature: many of the issues are significant policy considerations that, for any change to be contemplated, require further detailed assessment.

Issue raised	Preliminary PHARMAC comment
<p>PHARMAC inevitably makes value judgements when funding.</p> <p>There is a large number of value judgements to choose from, some potentially conflicting (that is, ‘best health outcomes’ can mean different things).</p>	<p>This is the basis of PHARMAC’s nine decision criteria. This, in PHARMAC’s view, is a key reason why the same approach should be used to assess high cost medicines, as for other medicines. The same issues are at play, albeit with a different magnitude of cost. As noted by some commentators, the principles are relevant not just to high cost medicines but to all pharmaceutical funding.</p>
<p>PHARMAC needs to be more explicit and transparent about what value judgements are made.</p>	<p>While the decision criteria themselves are explicit, it is important– for the general acceptance of decisions – that PHARMAC’s decisions are also understood. PHARMAC is aware that some stakeholders would like more information regarding PHARMAC’s decisions. There are pros and cons with doing this that need careful assessment. As decisions will depend on a wide range of factors at any time, it is possible that decisions – if explained in significant detail - may be perceived as inconsistent when in fact the best decision in the circumstances was made. This is a broader issue than decisions related to high cost medicines (and, as noted above, is a complex issue requiring careful assessment).</p>
<p>‘Maximising value for money’ is at the heart of the issue. The question is “what does maximising value for money mean?”</p>	<p>Some of the commentators suggest that this does not necessarily mean the most quality-adjusted life years (QALYs) gained from the pharmaceutical budget. In terms of PHARMAC’s legislative objective, in trying to secure the “best health outcomes”, ‘best’ does not necessarily have to mean the most QALYs for the money spent. All nine decision criteria considered by the PHARMAC Board are important.</p>
<p>PHARMAC needs to consider the values of its stakeholders, and these are likely to change over time.</p>	<p>In essence, the issue is whether the trade-offs made by the PHARMAC Board accord with the trade-offs that stakeholders would make, and what the general public would find acceptable. Stakeholder interests, while well intentioned, may not always align with the public good which the decision-making process is intended to protect. It is PHARMAC’s role to represent the public interest. All PHARMAC’s funding decisions are consulted on, with responses considered by the Board – accordingly, the Board takes into account the views of interested parties. Other opportunities for engagement – such as this consultation paper – further help PHARMAC to understand stakeholder views.</p>
<p>Decisions invariably entail judgements by the decision-maker; matters are not always “black” and “white” or technically clear.</p>	<p>The need for judgement is a practical reality of decision making in a complex environment. The PHARMAC Board is appointed to make these judgements with the best available evidence and information available to it, including through consultation with interested parties. If decisions were highly mechanistic or formulaic, decision-making judgements would still be required to set the formulae or algorithms, which, in themselves, are less flexible and still involve judgements.</p>
<p>There should be greater clarity and explanation around PHARMAC’s Decision Criteria and how they are used.</p>	<p>This is a variation on the issues and comments above. Decision criteria periodically need to be reviewed, whether their content or how they are applied. For example, PHARMAC has recently consulted on the revision to its Prescription for Pharmacoeconomic Analysis, which contributes to assessment under Decision Criterion Five.</p>
<p>Cost utility analysis (CUA) is an important tool, but CUA in itself does not promote particular values.</p>	<p>PHARMAC agrees that CUA is a tool and not a value in itself. Further, such analysis is one of many important inputs into funding decisions.</p>
<p>PHARMAC should consider the</p>	<p>PHARMAC is always open to assessing new approaches to improve its systems</p>

Issue raised	Preliminary PHARMAC comment
use of Multiple Criteria Decision Analysis (MCDA) as a research tool (at this stage).	and processes and is aware of this tool. As this is about the application of decision criteria generally, it is not specifically a matter related to funding high cost medicines.
There were arguments for and against using the 'rule of rescue' as a principle for pharmaceutical funding.	'Rule of rescue' reflects a natural human instinct to helping people in peril regardless of cost. The range of arguments both for and against the use of this rule demonstrates the complexity of this issue. On one hand, helping people in greatest need is very understandable; on the other, the rule of rescue is not always underpinned by objective evidence to support action ahead of other alternatives.
Some commentators noted that the lead reports did not deal with the local New Zealand situation and Maori and Pacific philosophies/kaupapa. The general point seemed to be that the approach taken in New Zealand needs to take into account features specific to New Zealand.	The existing Decision Criteria allow the PHARMAC Board to take into account a wide range of factors relevant to decisions, including New Zealand specific factors. There is a particular decision criterion related to the impact on Maori and Pacific health. As well as its clinical advisory committee, PTAC, PHARMAC will seek input from its Consumer Advisory Committee (CAC). There is Maori representation across CAC, PTAC, PHARMAC's Board, and PHARMAC management and staff.
Mixed comments regarding an allocation advisory committee.	Such a committee would provide advice on value judgements and, in essence, what weight should be given to particular criteria in particular circumstances. This, however, is already the role of the PHARMAC Board. Such a committee may simply shift the decision making trade-offs to a separate body (the same issues in a different place, and possibly with additional administrative costs). Were that not the case, such a committee would have 'all rights and no responsibility' and it is difficult to see – given the role of the PHARMAC Board – how a separate committee (taking into account all other existing process steps) would add value to the decision making process.
Mixed comments regarding clarifying the process for reviewing PHARMAC decisions; some discussion about an appeal process.	This relates to PHARMAC's decision making generally, not specifically high cost medicines. PHARMAC's decisions are subject to judicial review. Whether there should be a merit-based appeals process as well has been extensively debated in the past, both with reference to PHARMAC and other government decision making bodies (without extension to the right of judicial review). Regardless of what review mechanisms exist, there will always be the need to make social choices with regard to which medicines to fund.
An argument was made for a stand-alone budget for some medicines (a so-called 'tithe approach'). Having such a budget could give comfort that at least some high cost medicines would be funded.	Even if a separate budget were established, all of the same issues related to funding high cost medicines would still exist (the same difficult judgements need to be made). The same problem remains, and does not answer at what level to set the 'tithe' and how this should be decided. Depending on its size, such a budget could also be used quickly given the cost of such medicines. Further, any stand-alone budget creates boundary issues, that is, incentives to choose a budget – for a funding application – best suited to commercial imperatives. A separate budget would also preclude PHARMAC from making funding trade-offs across all pharmaceuticals. This could mean that lower value investments are made from one budget than the other, with possible adverse equity implications.

Conclusion

Work to date, including input from independent experts, indicates there are no persuasive arguments for treating the funding of high cost medicines differently to other medicines, that is, the same analytical tools and decision making framework are appropriate. PHARMAC now wishes to test this view with other interested parties and is seeking public submissions by Monday 5 March 2007. Having reviewed submissions, PHARMAC will report on them and any further steps related to this work.

There are a number of ethical theories that can be applied to how high cost (or indeed any) medicines are assessed. How decisions are made will depend on the set of values used. The dialogue about social choices between decision makers and those affected by decisions does not, and should never, reach an end.

Appendix One

Social value judgements: principles for the development of NICE guidance. National Institute for Clinical Excellence, December 2005. URL: <http://www.nice.org.uk/page.aspx?o=283494>

Appendix Two

Authors of the two reports and nine reviews of those reports

1. Professor Raanan Gillon, emeritus professor of medical ethics at Imperial College, London, Chairman, Institute of Medical Ethics
2. Associate Professor Paul Hansen Department of Economics, University of Otago, Dunedin
3. Associate Professor Toni Ashton (School of Population Health, University of Auckland)
4. Sandra Coney (PHARMAC Consumer Advisory Committee)
5. Matiu Dickson (Ngaiterangi (Ngai Tukairangi); School of Law, University of Waikato; PHARMAC Consumer Advisory Committee)
6. Dr. David Hadorn (LECG; ex-US Panel on Cost Effectiveness in Medicine)
7. Dr. George Laking (Christie Hospital, Manchester)
8. Dr. Robert Logan (Hutt Valley District Health Board; ex-Chair National Health Committee)
9. Professor Nicholas Mays (London School of Hygiene & Tropical Medicine, University of London; New Zealand Treasury; co-editor Journal of Health Services Research & Policy)
10. Dr. Andrew Moore (Department of Philosophy, University of Otago; Chair National Ethics Advisory Committee; ex-National Health Committee)
11. Dr. Martin Wilkinson (School of Population Health, University of Auckland)

Appendix Three

The two full reports and nine reviews of those reports

All of these reports should be read together

1. Report no. 1 (Prof. Raanan Gillon)
2. Report no. 2 (Assoc. Prof. Paul Hansen)
3. Review no. 1 (Assoc. Prof. Toni Ashton)
4. Review no. 2 (Sandra Coney)
5. Review no. 3 (Matiu Dickson)
6. Review no. 4 (Dr. David Hadorn)
7. Review no. 5 (Dr. George Laking)
8. Review no. 6 (Dr. Robert Logan)
9. Review no. 7 (Prof. Nicholas Mays)
10. Review no. 8 (Dr. Andrew Moore)
11. Review no. 9 (Dr. Martin Wilkinson)