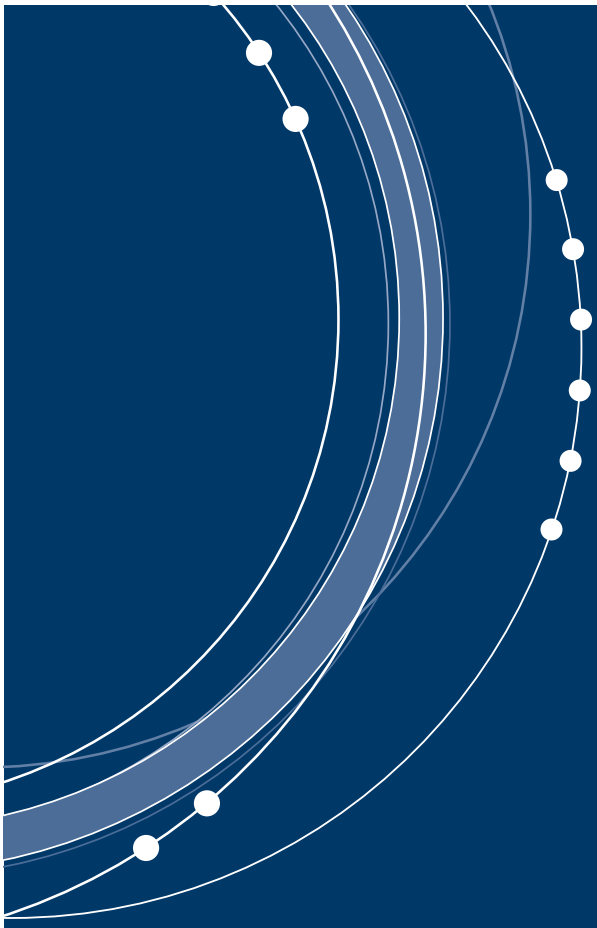

How should high cost medicines be funded?

Paper for public consultation

December 2006

Investing in Health

PHARMAC

Pharmaceutical Management Agency

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HOW SHOULD HIGH COST MEDICINES BE FUNDED?

PAPER FOR PUBLIC CONSULTATION
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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 approach[2]).

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; they have been managed by PHARMAC since 1993.

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 (PFPA), 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
PHARMAC inevitably makes value judgements when funding. There is a large number of value judgements to choose from, some potentially conflicting (that is, 'best health outcomes' can mean different things).	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.
PHARMAC needs to be more explicit and transparent about what value judgements are made.	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).
'Maximising value for money' is at the heart of the issue. The question is "what does maximising value for money mean?"	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.
PHARMAC needs to consider the values of its stakeholders, and these are likely to change over time.	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.
Decisions invariably entail judgements by the decision-maker; matters are not always "black" and "white" or technically clear.	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.
There should be greater clarity and explanation around PHARMAC's Decision Criteria and how they are used.	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.
Cost utility analysis (CUA) is an important tool, but CUA in itself does not promote particular values.	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.

PHARMAC should consider the use of Multiple Criteria Decision Analysis (MCDA) as a research tool (at this stage).	PHARMAC is always open to assessing new approaches to improve its systems 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

National Institute for Health and Clinical Excellence

SOCIAL VALUE JUDGEMENTS

Principles for the development of NICE guidance

Thursday 8th December 2005

Contents

Preface

These guidelines have been produced to help the Institute and its advisory bodies in developing NICE guidance. It is a 'living document' that will be updated to reflect developments within the academic world, the work of the Citizens Council, and the Institute's own emerging experience as it continues to develop guidance for the NHS and the wider public health community.

The guidelines will be formally reviewed in 2007 but earlier if the need arises. At that time we anticipate that important additional areas will be incorporated. These include the problem of comorbidity, the so-called "rule of rescue", approaches to the trade-off between risk and benefit within the interventional procedures programme, and the question of how NICE should approach the requirement to foster innovation. In addition, future editions will examine the social value judgements that should inform NICE's public health guidance.

Summary of principles

These guidelines describe the social value judgements that should, generally, be incorporated into the processes used to develop NICE guidance and be applied when preparing individual items of NICE guidance. The Institute recognises, however, that there will be circumstances when – for valid reasons – departures from these general principles are appropriate. When departures from these principles are made, the reasons should be explained (section 1.2).

Principle 1

The fundamental principles that underpin the processes by which NICE guidance is developed should be maintained for current, and applied to future, forms of guidance (section 3.3).

Principle 2

For both legal and bioethical reasons those undertaking technology appraisals and developing clinical guidelines must take account of economic considerations (sections 4.1 and 6.1).

Principle 3

NICE guidance should not support the use of interventions[1] for which evidence of clinical effectiveness is either absent or too weak for reasonable conclusions to be reached (section 4.1).

Principle 4

In the economic evaluation of particular interventions, cost–utility analysis is necessary but should not be the sole basis for decisions on cost effectiveness (section 4.1).

Principle 5

NICE guidance should explain, explicitly, reasons for recommending – as cost effective – those interventions with an incremental cost-effectiveness ratio in excess of £20,000 to £30,000 per QALY (section 4.3).

Principle 6

NICE clinical guidance should only recommend the use of a therapeutic or preventive intervention for a particular age group when there is clear evidence of differences in the clinical effectiveness of the measure in different age groups that cannot be identified by any other means (section 5.1).

[1] The term 'intervention' is used in these guidelines to encompass health technologies and any other measure used to influence the course of a particular condition.

Principle 7

In setting priorities there is no case for the Institute or its advisory bodies to distinguish between individuals on the basis of gender or sexual orientation unless these are indicators for the benefits or risks of preventative or therapeutic interventions (section 5.2).

Principle 8

In developing clinical guidance for the NHS, no priority should be given based on individuals' income, social class or position in life and individuals' social roles, at different ages, when considering cost effectiveness. Nevertheless, in developing its approach to public health guidance, NICE wishes its advisory bodies to promote preventative measures likely to reduce those health inequalities that are associated with socioeconomic status (section 5.3).

Principle 9

NICE clinical guidance should only recommend the use of an intervention for a particular racial (ethnic) group if there is clear evidence of differences between racial (ethnic) groups in the clinical effectiveness of the intervention that cannot be identified by any other means (section 5.4).

Principle 10

NICE and its advisory bodies should avoid denying care to patients with conditions that are, or may be, self-inflicted (in part or in whole). If, however, self-inflicted cause(s) of the condition influence the clinical or cost effectiveness of the use of an intervention, it may be appropriate to take this into account (section 5.5).

Principle 11

Although respect for autonomy, and individual choice, are important for the NHS and its users, they should not have the consequence of promoting the use of interventions that are not clinically and/or cost effective (section 5.6).

Principle 12

It is incumbent on the Institute and its advisory bodies to respond appropriately to the comments of stakeholders and consultees and, where necessary, to amend the guidance (section 5.7).

The board is aware, however, that there may be occasions when attempts are made (directly or indirectly) to influence the decisions of its advisory bodies that are not in the broad public interest. The board requires the Institute, and members of its advisory bodies, to resist such pressures (section 5.7).

Principle 13

Priority for patients with conditions associated with social stigma should only be considered if the additional psychological burdens have not been adequately taken into account in the cost–utility analyses (section 6.5).

1 Introduction

1.1 Background

When developing advice to the National Health Service (NHS) and, since 1 April 2005 to the wider public health community, the Institute bases its conclusions on the 'best available' evidence. The best available evidence is not always very good and is rarely (if ever) complete. It may be of poor quality, lack critical elements, or both. Those responsible for formulating the Institute's advice about efficacy, effectiveness, cost effectiveness and safety are therefore inevitably required to make judgements.

These judgements fall broadly into two categories. Scientific value judgements are concerned with interpreting the significance of the available scientific, technical and clinical data. Social value judgements relate to society rather than to basic or clinical science: they take account of the ethical principles, preferences, culture and aspirations that should underpin the nature and extent of the care provided by the NHS. Nevertheless, the distinction is not absolute: there is a scientific dimension to the measurement and understanding of social value judgements, but this does not form part of this document.

1.2 Aim of these guidelines

This document describes the Institute's approach to incorporating social value judgements into the processes used to develop NICE guidance, as well as the principles that should be applied in developing individual items of guidance. It is primarily, though not exclusively, concerned with those social value judgements that are involved in developing conclusions about cost effectiveness and particularly those judgements that have implications for priority setting and resource allocation.

The Institute recognises, however, that there will be circumstances when – for valid reasons – departures from these general principles are appropriate. When this happens, however, the Institute expects the reasons for doing so to be explained in the guidance.

1.3 Areas outside the remit of these guidelines

There are four important areas that are not included in these guidelines.

Firstly, the guidelines do not cover the social value judgements required in evaluating the balance between the risks and benefits of interventional procedures. The Institute intends to include such considerations in future editions of the guidelines. They have been omitted from this document, partly because the bioethical literature is generally weak in the area and partly because the Institute has not as yet undertaken (or commissioned) work relating to this topic.

Secondly, the guidelines do not, in the main, consider the social value judgements concerned with developing public health advice. This issue will also be addressed in future editions.

Thirdly, the guidelines do not describe the social value judgements that should be applied in developing guidance on the use of 'ultra-orphan'[2] health technologies. NICE is currently formulating its position on the appraisal of these technologies and its conclusions are not yet available.

Finally, the guidelines do not cover the social value judgements required of those undertaking the National Confidential Enquiries (1) because on 1 April 2005 the National Patient Safety Agency took over responsibility for this programme.

1.4 Intended audiences for these guidelines

These guidelines are intended for three audiences.

They are addressed at those involved in constructing, or revising, the processes and procedures that determine the way NICE guidance is developed.

The guidelines are especially relevant to the Institute's advisory bodies (see the glossary) that are responsible for developing individual forms of NICE guidance.

They also attempt to help the Institute's stakeholders[3] and the wider public, to understand the social values that underpin NICE guidance.

1.5 Who has developed these guidelines?

Although the Institute's board takes ultimate responsibility for the content of all NICE guidance, senior members of the Institute's staff have devolved powers to approve documents on the board's behalf. These guidelines, however, are unusual in being the direct responsibility of the board. They take account (as described below) of the views of the Institute's advisory bodies and, in particular, of advice from its Citizens Council. They have also been revised in the light of comments on an earlier draft from a wide range of interested groups and individuals.

1.6 Methods used to develop these guidelines

These guidelines are based, primarily, on evidence from three sources – the published literature, two reports of the Citizens Council (2, 3), and the results of a survey (4, 5) conducted on behalf of the Institute.

[2] So-called 'ultra-orphan' health technologies are defined by NICE as those used to treat conditions with a prevalence of less than 1 in 50,000 in the UK.
[3] The Institute's stakeholders include professional bodies, industries, patients/carers and their representative organisations relevant to particular forms of NICE guidance.

1.6.1 The literature

The aim of the review of the literature was to identify, and synthesise, published material that might inform the board's approach to developing its social value judgements. Publications relating to three particular areas were sought:

- general principles of bioethics
- bioethical considerations of resource allocation and priority setting
- reports of relevant studies of professional and public attitudes to resource allocation, priority setting and rationing.

Publications relating to all three areas were identified from the peer-reviewed literature (through Medline, Embase, Science Citation Index, Social Science Citation Index, Arts and Humanities Citation Index), books and monographs, as well as the 'grey' literature.

1.6.2 The Citizens Council

The members of NICE's advisory bodies are appointed for their competence in making scientific value judgements but neither they, nor the board, can legitimately impose their own social value judgements on the NHS and the patients that it seeks to serve.

The Institute therefore established the Citizens Council as a formal committee of the Institute to help develop the broad social values that NICE should adopt in preparing its guidance. The 30 members of the Council reflect the age, gender, socioeconomic status and ethnicity of the people of England and Wales. Councillors serve for a period of 3 years, with one third retiring each year. They do not represent any particular section or sector of society; rather, they bring their own personal attitudes, preferences, beliefs and prejudices (6). They and their families have experience of the NHS as patients, but none of the members is a healthcare professional.

At each meeting, the Council is asked for its views on an issue about which the Institute seeks advice. Meetings are facilitated by an independent organisation and members have the opportunity to hear, and cross-examine, expert witnesses as well as to engage in discussion and deliberation in both plenary and small-group sessions. The Council's conclusions are contained in a report that is presented to the Institute's board.

1.6.3 The ICM survey

In March 2004 a telephone survey was conducted by ICM, on behalf of NICE, amongst a sample of 1010 people in the UK. The questions related to:

- awareness of the existence and functions of the Institute (4)
- attitudes about priority setting, particularly in relation to patients' age (5).

The ICM survey is a useful source of information about the public's perceptions, but NICE recognises that polling, like all methods for seeking 'public opinion', has two major limitations. Firstly, the results are very sensitive to the way questions are 'framed'. Secondly, responses are instantaneous replies without the benefit, necessarily, of learning about the underlying

issues. NICE therefore triangulates the results of polling data with the reports of the Citizens Council and the relevant literature.

More information about the ICM poll can be found at www.nice.org.uk/page.aspx?o=268902.

2 Principles of bioethics

2.1 Moral principles

The Institute subscribes to the widely accepted moral principles (7–11) that are expected to underpin clinical and public health practice:

- respect for autonomy
- non-maleficence
- beneficence
- distributive justice.

These so-called 'four principles' have been adopted by NICE because they provide a simple, accessible, and culturally neutral approach that encompasses most of the moral issues that arise in healthcare (12). In accepting these principles, the Institute also recognises (7) that there are tensions both within and between them; it also accepts that no one principle has an overriding priority over another. Indeed, these guidelines are, to a considerable extent, concerned with attempting to resolve the inherent tensions between these moral principles within the context of the social value judgements that the Institute and its advisory bodies have to make.

2.1.1 Respect for autonomy

Respect for autonomy acknowledges the rights of individuals to make informed choices in relation to healthcare, health promotion and health protection. It is inherent in the concept of 'patient choice'. It cannot, though, be applied universally. By virtue of mental or physical incapacity, for example, some people may be unable to make informed choices; public health measures must sometimes be necessarily imposed on whole populations (such as smoking bans in enclosed spaces); and providing a few people with very expensive treatments, on the basis of personal preference, could deprive many others of more cost-effective interventions.

2.1.2 Non-maleficence

Non-maleficence asserts an obligation not to inflict damage (either physical or psychological) and has often been associated with the maxim 'first, do no harm'. Yet no intervention[4] is free from the potential to cause adverse consequences: it is the balance between the benefits and harms that determines (at least in part) whether an intervention is appropriate.

2.1.3 Beneficence

Beneficence, which is closely related to non-maleficence, refers to the obligation to benefit individuals. Moral philosophers distinguish between 'positive beneficence' and 'utility', with the former describing benefits that can be accrued and the latter attempting to balance benefits and harms. Yet no clinical or public health intervention is invariably beneficial for everyone; and,

[4] The term 'intervention' is used in these guidelines to encompass health technologies and any other measure used to influence the course of a particular condition.

in the context of the work of NICE, it is respect for utility that is usually more relevant.

2.1.4 Distributive justice

'Distributive justice', as it relates to healthcare, is concerned with the provision of services in a fair and appropriate manner in the light of what is due, or owed, to people. Problems of distributive justice have become particularly manifested in healthcare, because of the universal mismatch between demands and resources. It is one of the most debated topics in bioethics, and is probably the most culture-specific.

The traditional, paternalistic approach to distributive justice has been the 'need principle'[5]. This claims that priorities should be set solely on the basis of clinical need as defined by the current degree of ill health (13); in practice, the need principle has largely been based on the premise 'doctor knows best'. This principle, however, takes no account of other issues and provides no solution to problems relating to a healthcare system as a whole. The need principle, as defined here, has some relevance to the 'rule of rescue' but is not otherwise considered further. Of the other main theories of distributive justice (7), two are especially relevant to the British healthcare system[6]: utilitarianism and egalitarianism.

Utilitarianism[7] seeks to maximise overall good (or public utility). In its purest form, the utilitarian approach considers distributive justice to be best served by maximising social utility. It is often expressed, in shorthand, as 'the greatest good for the greatest number'.

Egalitarianism seeks fairness either in equality of opportunity (the 'fair opportunity' rule) or in equality of outcomes. Egalitarians seek healthcare to be distributed, so far as is possible, so that each person can achieve a fair share of the opportunities available in the particular society.

There is no consensus about which of these (and other) competing theories of distributive justice most appropriately captures the bioethical basis of the fair allocation of healthcare resources (7,13,14). Utilitarianism can allow the interests of minorities to be overridden by the majority, and it has little or nothing to offer in eradicating health inequalities. On the other hand, the notion of 'utility' places a premium on the 'efficiency' of a healthcare system, and provides a compelling warning about the dangers of ignoring opportunity costs (section 4). Egalitarianism emphasises fairness, and access to an adequate (though not necessarily maximum) level of healthcare. Its

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[5] The 'need principle' is sometimes described as the Marxist approach to distributive justice.

[6] A third, widely held theory of distributive justice is 'libertarianism'. This espouses a free-market solution for the distribution of healthcare. Since, in the UK, over 90% of all healthcare is provided through the NHS and funded from general taxation, libertarianism is irrelevant to these guidelines.

[7] Since classical utilitarianism is concerned with maximising happiness (a concept which is not concerned solely with health) some (11) prefer to describe the approach, in healthcare, as the 'maximising principle' but the term 'utilitarianism' will be retained in this discussion.

implications, though, remain uncertain (7). There is lack of clarity both in the definition of 'adequate' healthcare, and in the distinctions between what is fair and unfair or between what is unfair and what is unfortunate.

Both theories of distributive justice clash, at some point, with the considered moral convictions of most people. Each, however, articulates ideas that most would be reluctant to relinquish; and where one theory is weak, the other is often strong and some compromise has to be found (9). This is uncomfortable and has been described, by one commentator (15), as 'muddling through elegantly'.

This compromise is not a synthesis of the conflicting demands of utilitarianism and egalitarianism but a procedural device that allows the resolution of divergent values in order to provide 'accountability for reasonableness'. Decisions about social value judgements that are either implicit, or explicit, in NICE guidance should focus on the acceptable implications of each theory of distributive justice without necessarily, or invariably, choosing one over another. Inevitably, some people will be dissatisfied (16), for not everyone's claims will be met. Procedural justice, however, places a premium on ensuring that the processes by which decisions are reached have legitimacy (14, 17–20).

2.2 Strategies for setting priorities

There is a groundswell of opinion amongst bioethicists and political philosophers that, if there is to be confidence in the legitimacy of decisions, the procedures adopted should have all four of the following characteristics:

- publicity
- relevance
- revision and appeals
- regulation.

2.2.1 Publicity

Decisions about limits on the allocation of resources must be public. This includes not only the decisions themselves, but also the grounds for making them. It does not, however, require that all the criteria for decision-making should be established in advance: rather, there should be room for the development of 'case-law'.

2.2.2 Relevance

'Relevance' means that the grounds for decisions are ones that fair-minded people would agree are relevant to meeting healthcare needs, especially when there are constraints on resources. In particular, 'relevance' focuses on the importance of deliberation about the limits of the common good and acknowledges that such 'deliberative democracy' should involve both the decision-makers themselves and those whom the decisions may affect.

2.2.3 Revision and appeals

There must be opportunities for challenging decisions and mechanisms for resolving disputes. There should be systems in place for revising decisions when new, or additional, evidence becomes available or new arguments are put forward.

2.2.4 Regulation

There should be either voluntary or public regulation of the process of decision-making to ensure that it has all three of the above characteristics (publicity, relevance and opportunities for revisions and appeals).

2.2.5 Accountability for reasonableness in decision-making

Ensuring that procedures have all four of these characteristics makes decision-makers 'accountable for their reasonableness' (14, 17–20) (see section 2.1.4). Critics (7) claim that majority preferences – however well informed and fair – will sometimes lead to unjust outcomes, that deliberative democracy in action will 'most certainly' conflict with the principles of justice, and that 'deep suspicion is warranted about procedural strategies for setting priorities'. Such criticisms have some merit: yet no reasonable theoretical or practical alternatives have been proposed to resolve the conflicting theories of distributive justice.

For the NHS, there is a further reason for ensuring broad 'accountability for reasonableness' (14). The NHS is constructed on the principle of social solidarity. It provides healthcare for all UK citizens, at times of need, and irrespective of their ability to pay. Because the NHS is funded from general taxation, it must be right for UK citizens to have the opportunity to be engaged in the broad principles by which the NHS's priorities are set. They are, after all, the ultimate providers – through their taxes – of the services that are available.

3 Applying principles through process

3.1 The Institute's guidance programmes

The National Institute for Clinical Excellence (NICE) was established in 1999 to offer NHS healthcare professionals guidance on providing their patients with the highest attainable standards of care[8]. It has done this through three major programmes (1).

3.1.1 Health technology appraisals programme

The Institute advises on the use of individual, or classes of, health technologies. These include pharmaceuticals, devices, diagnostics, surgical and other procedures, and health promotion techniques. NICE's advice is based on considerations of both the clinical and cost effectiveness of the particular technology (or class of technologies) under examination.

3.1.2 Clinical guidelines programme

The Institute advises on the clinical management of specific conditions or disorders by developing and disseminating 'clinical guidelines' defined as: 'Systematically developed statements to assist practitioner and patient decisions about appropriate healthcare for specific clinical circumstances'. As with its appraisals of health technologies, NICE's clinical guidelines take account of both the clinical and cost effectiveness of various clinical management options.

3.1.3 Interventional procedures programme

The Institute advises on the safety and efficacy of new and established interventional procedures for use within the NHS. The Institute defines interventional procedures as: 'those used for diagnosis or treatment that involve incision, puncture, entry into a body cavity or the use of ionising, electromagnetic or acoustic energy'.

In its assessment of interventional procedures, NICE takes account of the efficacy (rather than effectiveness) of the procedure and whether its safety (in relation to its efficacy) is compatible with its benefits. This programme, unlike the appraisals and clinical guidelines programmes, is not concerned with cost effectiveness although procedures that are regarded as safe and efficacious may undergo subsequent appraisals.

[8] The National Institute for Clinical Excellence (NICE) was renamed on 1 April 2005, when it took on the functions of the Health Development Agency and became the National Institute for Health and Clinical Excellence. The organisation will produce public health guidance (on the promotion of good health and the prevention of ill health) for those working in the NHS, local authorities and the wider public and voluntary sector as well as the clinical guidance that it has been developing since its establishment in 1999. However, this document deals only with the clinical guidance for which NICE is responsible.

3.2 Legal requirements underpinning NICE guidance

In developing its guidance, NICE is obliged, by law, to conform to the provisions laid out in its Establishment Orders (21–23), and in its Directions from the Secretary of State for Health (24).

3.2.1 NICE's Establishment Orders

The Institute's original Establishment Order (21) provided that: 'The Institute shall perform such functions in connection with the promotion of clinical excellence in the health service as the Secretary of State may direct'. To avoid any misunderstanding about whether economic considerations should be taken into account, the Order was later amended (22) to: 'The Institute shall perform such functions in connection with the promotion of clinical excellence **and with the efficient use of available resources** in the health service as the Secretary of State may direct'.

The Institute is thus legally obliged to take account of both clinical and cost effectiveness in developing guidance in its technology appraisals and clinical guidelines programmes. The Establishment Order restricts the perspective of NICE's clinical guidance to the NHS and personal social services (PSS). In developing its public health guidance, however, the Institute's perspective has been extended to include 'other available public funds' as well as the NHS and PSS (24). This recognises the fact that responsibility for implementing public health measures extends beyond the NHS (for example, to local authorities). These 'cost-effectiveness' provisions do not apply to NICE's interventional procedures programme, which covers only safety and efficacy.

3.2.2 Directions to the Institute

The Secretary of State's Directions (24) to NICE require that, in appraising the clinical benefits and the costs of interventions, the Institute should have regard to the following factors.

1 The broad balance of benefits and costs.
2 The degree of clinical need of patients with the condition or disease under consideration.
3 Any guidance issued to the NHS by the Secretary of State that is specifically drawn to the attention of the Institute by the Secretary of State and any guidance issued by the Secretary of State.
4 The potential for long-term benefits to the NHS of innovation.

3.3 Essential features of NICE guidance

The Institute has adopted, and published, process documents for each form of NICE guidance (25–27). Although, inevitably, the process documents for each programme differ in detail they all have common features:

- methodological robustness
- inclusiveness
- transparency
- independence
- appeals
- review.

NICE guidance should also be timely. This means that it should be available at a time when it is of greatest use to its intended audiences.

3.3.1 Methodological robustness

The Institute's guidance is invariably based on a systematic review of the relevant published, and unpublished, literature. Although NICE is prepared to accept unpublished data it does so with reluctance: it believes that the data on which its guidance is based should be in the public domain (that is, accessible in print or electronic formats). Only in this way can there be professional and public confidence in its guidance. Nevertheless, refusal to accept unpublished data might disadvantage patients and breach the principles of non-maleficence and beneficence. In addition, rejection of relevant data would probably be unacceptable to the Courts.

3.3.2 Inclusiveness

The development of NICE guidance involves all those who have, or might have, an interest as either 'consultees' or 'commentators' (for technology appraisals) or 'stakeholders' (for clinical guidelines). These include relevant professional bodies, patients and patient–carer organisations, and (in the case of manufactured technologies) healthcare industries. All are involved with determining, at the start of the process, the scope of the guidance; all have an opportunity to comment on initial drafts of guidance; and all have the opportunity to make representations about the proposed final version of the guidance.

3.3.3 Transparency

The documentation supporting all NICE guidance is freely available on the Institute's website apart from data submitted as 'commercial in confidence' or 'academic in confidence'. Initial and final drafts of all forms of guidance are published, and interested parties may comment even if they are not registered as stakeholders or consultees. NICE guidance attempts to explain the reasons for the Institute's advice and the interpretation that its advisory bodies have placed on the available data.

3.3.4 Independence

All NICE guidance is developed by the independent members of the Institute's advisory committees (the appraisals committee and the interventional procedures advisory committee) and guideline development groups. They are drawn from the NHS, academia, the industries and patient–carer organisations. Their conclusions are reached only after extensive deliberation.

All members of the Institute's board, its staff, and members of its advisory bodies are required to make annual declarations (25) of any financial links they may have with the pharmaceutical, devices or diagnostics industries (19). Members of the board and advisory bodies are asked, in addition, to declare again their relevant interests at the start of each agenda item in a meeting (28).

3.3.5 Appeals

All three programmes provide consultees and stakeholders with the opportunity to comment on draft forms of NICE guidance. In the technology appraisals programme, consultees have additional rights of appeal to a panel appointed by the board (25). As the Institute is a public body, its guidance can be challenged in the UK (and EU) courts.

3.3.6 Review

When it is published, each of NICE's appraisals and clinical guidelines has a review date, which is the date at which NICE's Guidance Executive will consider the options for review of the guidance. These review dates are usually 3 and 4 years after publication (for appraisals and clinical guidelines, respectively) but if significant new data are anticipated or emerge the review dates may be brought forward.

3.3.7 Implementation

At the time NICE was proposed (29) its role did not include responsibility for implementing the guidance it produced. Recently, however, the Institute's Directions have been amended to provide a legal framework for an implementation programme (24). A number of practical measures have been introduced to support the implementation of NICE guidance. First, NHS trusts have a legal obligation (30) to make available resources to provide patients with technologies recommended in NICE's appraisals guidance. Secondly, NICE guidance is highlighted in the Department of Health's outline (31) of the standards that patients can expect to receive from the NHS. Thirdly, the Institute itself has established an implementation programme (32).

3.3.8 Legitimacy

These arrangements, collectively, offer 'accountability for reasonableness' (15, 17–20) (see section 2.1.4). They provide NICE guidance with a legitimacy that would otherwise be lacking, and have been commended on both scientific and technical (33), as well as political (34), grounds. Daniels and Sabin (12) acknowledge that NICE's processes embody key elements of accountability for reasonableness although they have reservations about the Institute's engagement with the public[9]. Empirical evidence is now emerging to suggest that this approach is acceptable to the public (35).

The principles that underpin the processes by which NICE guidance is currently developed should, therefore, be maintained and applied to future forms of advice (for example, public health guidance).

Principle 1

The fundamental principles that underpin the processes by which NICE guidance is developed should be maintained for current, and applied to future, forms of guidance.

[9] At the time that Daniels' and Sabin's comments were made, the Citizens Council had been planned (32) but not yet implemented.

4 Cost effectiveness and setting priorities

4.1 The Institute's approach to economic evaluation

The Institute accepts that, for both legal and bioethical reasons, in undertaking technology appraisals and developing clinical guidelines it must take account of economic considerations. Decisions about the total resources available for healthcare are, rightly, the responsibility of parliament and inevitably compete with other demands such as education, defence and transport. Within the allocations made by parliament, the resources for the NHS are finite, and the use of cost-ineffective interventions in one area of practice will deny the availability of cost-effective interventions in another. The Institute thus recognises that both it, and its advisory bodies, have a responsibility to avoid issuing guidance that would incur 'opportunity costs' that would lead to the substitution of one form of inequality by another one.

Principle 2

For both legal and bioethical reasons those undertaking technology appraisals and developing clinical guidelines must take account of economic considerations.

Economic evaluation in healthcare requires that the particular intervention under consideration has been shown to be clinically effective. Although there are various ways in which clinical effectiveness can be established, the requirement to do so is not only intellectually compelling and essential for a quantitative approach to economic evaluation, but also strongly supported by the British public (5). The Institute recognises the distinction between 'evidence of lack of effectiveness' and 'lack of evidence of effectiveness', but nevertheless considers that, in general, NICE and its advisory bodies should avoid promoting the use of interventions for which evidence of clinical effectiveness is either absent or is too weak for reasonable conclusions to be reached. This expectation should not, however, be overinterpreted. There will be circumstances, particularly where evidence is weak or entirely lacking, where judgement and experience strongly suggest that particular strategies (such as 'good clinical practice') provide patients with benefits in a cost-effective manner.

Principle 3

NICE guidance should not support the use of interventions for which evidence of clinical effectiveness is either absent or too weak for reasonable conclusions to be reached.

The Institute's preferred approach (25, 37) to the economic evaluation of clinical interventions is cost–utility analysis. In developing its clinical guidance, NICE is required (section 3.2.1) to confine its estimation of costs to those falling on the NHS and PSS. In its public health guidance, however, the

Institute is expected to expand the cost base to include other available public funds as well as those of the NHS and PSS. The principal measure of health outcome adopted by the Institute is the quality-adjusted life year (QALY). This embodies the important social value judgement that to count only gains in life expectancy, without considering the quality of the additional life years, omits important dimensions of human welfare (37).

Value judgements embodied in health-related quality-of-life measures can be reasonably captured in terms of:

- physical mobility
- ability to self-care
- ability to carry out activities of daily living
- absence of pain and discomfort, and
- absence of anxiety and depression.

There are also value judgements in the ways in which these elements are combined (37) and the scoring given to the various combinations of levels of functioning.

The use of cost–utility analysis in resource allocation has aroused a substantial debate (7–9, 38–43). Charges of discrimination against children, elderly and disabled people, and people who are terminally ill, have led some to conclude that the use of QALYs leads to impermissible trade-offs in setting priorities. Nevertheless, most bioethicists and political philosophers are generally prepared to accept cost–utility analyses provided that they are used to inform, rather than direct, decisions about setting priorities, and that other considerations are available to constrain morally offensive trade-offs (7, 13, 14). The Institute's own position is that while it endorses the use of cost–utility analysis in the economic evaluation of particular interventions, such information is a necessary, but not sufficient, basis for decision-making.

Suggestions have been made that social value judgements in cost–utility analyses could be identified empirically, and embedded within mathematical models (43, 44). Equity weights for age and gender are, for example, included in the calculation of disability-adjusted life years (DALYs) (45). Equity weighting, though intellectually attractive, is premature in the light of the available evidence (43, 44). NICE does not include equity weighting in its approach to cost–utility analysis (25) but does not exclude the possibility for the future.

Principle 4

In the economic evaluation of particular interventions, cost–utility analysis is necessary but should not be the sole basis for decisions on cost effectiveness.

4.2 Efficiency versus equity

The tensions between the utilitarian and egalitarian theories of distributive justice (section 2) are reflected in the debate amongst health economists about the balance between efficiency and equity (46).

Cost–utility analyses provide measures of health benefits in terms of anticipated health gains, and their associated costs are assessed from estimates of resource expenditure. The goal is utilitarian: it seeks to ensure the greatest health benefits for the money expended; it unashamedly attempts to achieve efficiency. In its strictest interpretation it expounds a value judgement that seeks the most 'efficient' use of the resources available to the NHS and prizes the maximisation of the overall health of the population above all else. The limitations of the quest for pure efficiency were, for example, apparent in the initial (draft) prioritised list of healthcare services in the Oregon scheme (47). This was based on a rank order of cost utilities but produced unacceptable trade-offs: tooth-capping, for example, was ranked above emergency surgery for both acute appendicitis and ectopic pregnancy.

The Institute's rejection of both a strictly utilitarian (efficiency) approach to the economic evaluation of healthcare interventions and quantitative attempts to incorporate equity weighting into estimates of QALYs has important implications. Firstly, despite the Institute's rejection of efficiency as the sole criterion for deciding cost effectiveness, NICE and its advisory bodies nevertheless require some indication of the range of cost per QALY values that are acceptable. Without such information, inconsistencies between different forms of NICE guidance, and different advisory bodies, would be inevitable. Secondly, if it is accepted that the Institute and its advisory bodies should have latitude in their interpretation of the cost effectiveness of particular interventions, some indication of the nature of the social value judgements they should adopt is necessary. Guidance on the nature of the social value judgements that should be adopted is essential to ensure fairness as well as, again, to avoid inconsistencies between the decisions of different advisory bodies (or even the same body on different occasions).

4.3 Limits to cost effectiveness

Where one intervention appears to be more effective than another, the Institute and its advisory bodies have to determine whether the increase in cost associated with the increase in effectiveness represents reasonable 'value for money'. This is generally done by calculating the incremental cost-effectiveness ratio. For the reasons already stated (see section 4.1) the preferred approach is the cost (£) per QALY (33), although in some instances it has been necessary to use the cost (£) per life year gained or (particularly for anti-cancer drugs) the cost (£) per disease-free life year.

There is no empirical basis for assigning a particular value (or values) to the cut-off between cost effectiveness and cost ineffectiveness (37). The consensus amongst the Institute's economic advisors is that the Institute should, generally, accept as cost effective those interventions with an incremental cost-effectiveness ratio of less than £20,000 per QALY and that

there should be increasingly strong reasons for accepting as cost effective interventions with an incremental cost-effectiveness ratio of over £30,000 per QALY. These reasons (25,37) include the degree of uncertainty surrounding the estimate of the incremental cost-effectiveness ratio and, where appropriate, reference to previous appraisals. The Institute and its advisory bodies will also wish to consider social value judgements, including consideration of the nature of the condition, the particular patient population, and the intervention itself. These are discussed in separate sections of these guidelines (section 5).

The Institute is reassured by independent evidence (48, 49) that its advisory bodies have not adopted a rigid incremental cost per QALY 'threshold'. NICE is aware, however, that some commentators have criticised the Institute's range of acceptable incremental cost-effectiveness ratios as too generous. Williams (45), for example, has suggested that the 'common sense' approach would be to base the incremental cost-effectiveness ratio on the per capita gross domestic product. This, in the context of the UK, would represent an incremental cost per QALY 'threshold' value of £18,000 per QALY (50).

Suggestions such as these, however, rely on 'judgements' that carry no more (or less) authority than the collective judgement of the Institute's economic advisors. The Institute therefore wishes its advisory bodies to continue with the current range of acceptable incremental cost-effectiveness ratios, albeit with two provisos: first, that advisory bodies should explain, explicitly, their reasons for recommending – as cost effective – those interventions with an incremental cost-effectiveness ratio in excess of £20,000–£30,000 per QALY; and, second, that NICE will review this in the light of research currently being conducted through the NHS Research and Development's Methodology Programme.

Principle 5

NICE guidance should explain, explicitly, reasons for recommending – as cost effective – those interventions with an incremental cost-effectiveness ratio in excess of £20,000 to £30,000 per QALY.

5 Social value judgements – service users

The NHS seeks to provide comprehensive healthcare for the population of the UK that is free at the point of need. Patients should not be denied access to NHS treatment simply because of their age, disability, faith, gender, sexual orientation, socioeconomic status or race, because their illness may be self-inflicted, or because of some other 'non-health indicator'. However, for reasons previously discussed, on both bioethical and economic grounds, limits have to be placed on healthcare provision that take account of both efficiency and equity.

The board is conscious that discrimination can sometimes occur inadvertently (51) and asks the Institute and its advisory bodies to be especially vigilant in avoiding all forms of discrimination.

5.1 Age

- The issue of whether, or how, an individual's age should be taken into account in allocating healthcare resources has roused considerable debate.

NICE's Citizens Council concluded that (3):

- health should not be valued more highly in some age groups than in others
- individuals' social roles, at different ages, should not influence considerations of cost effectiveness
- however, where age is an indicator of benefit or risk, it is appropriate to take it into account.

The Institute's general principle is that patients should not be denied NHS treatment simply because of their age. NICE acknowledges that treatments can produce different benefits at different ages and that age itself may be the only identifiable indicator. Nonetheless, wherever practical, NICE's advisory bodies should avoid issuing guidance that refers to age if this is being used as a presumed proxy for some aspect of patients' health status.

Where NICE guidance refers to age it should only occur when all the following conditions are met:

- the evidence indicates that age is a good proxy for some aspect of patients' health status and/or the likelihood of adverse effects of the treatment, and
- there is no practical way of identifying patients other than by their age (there is, for example, no routinely available diagnostic test to measure the relevant aspect of their health status), and
- it is logically and/or biologically plausible that, because of their age, patients will respond differently to the treatment in question.

In such instances NICE and its advisory bodies should explain within the guidance the reasons for using age as an indicator. The use of arbitrary age

cut-offs intended to indicate (for example) 'old age', 'childhood' or 'adolescence' should be avoided. Where it is necessary to indicate an age cut-off, and where the treatment is appropriate only for people in a particular age group, then a reason for using this specific cut-off should be provided.

Principle 6

NICE clinical guidance should only recommend the use of a therapeutic or preventive measure for a particular age group when there is clear evidence of differences in the clinical effectiveness of the measure in different age groups that cannot be identified by any other means.

5.2 Gender and sexual orientation

Principle 7

In setting priorities there is no case for the Institute or its advisory bodies to distinguish between individuals on the basis of gender or sexual orientation unless these are indicators for the benefits or risks of preventative or therapeutic interventions.

5.3 Socioeconomic status

The Citizens Council considered that no priority should be given based on individuals' income, social class or position in life (2). Nor did the Council consider that individuals' social roles, at different ages, should influence considerations of cost effectiveness (2, 3).

The Institute supports these conclusions, as they relate to NICE's clinical guidance, and wishes its advisory bodies to take note of them in developing advice for the NHS. Nevertheless, in developing its approach to public health guidance, the Institute wishes its advisory bodies to promote preventative measures likely to reduce those health inequalities that are associated with socioeconomic status.

Principle 8

In developing clinical guidance for the NHS, no priority should be given based on individuals' income, social class or position in life, and individuals' social roles, at different ages, should not influence considerations of cost effectiveness. Nevertheless, in developing its approach to public health guidance, NICE wishes its advisory bodies to promote preventative measures likely to reduce those health inequalities that are associated with socioeconomic status.

5.4 Race (ethnicity)

There is no general case for limiting healthcare on racial (ethnic) grounds (51). NICE clinical guidance should only recommend the use of an intervention for a particular racial (ethnic) group if there is clear evidence of

differences between the groups in the clinical effectiveness of the intervention that cannot be identified by any other means. For example, it would be acceptable to restrict the use of the combination of hydralazine and isosorbide dinitrate (BiDi) for heart failure to Afro-Caribbeans, because of the absence of benefit in other ethnic groups (53).

Principle 9

NICE clinical guidance should only recommend the use of an intervention for a particular racial (ethnic) group if there is clear evidence of differences between racial (ethnic) groups in the clinical effectiveness of the intervention that cannot be identified by any other means.

5.5 Self-inflicted conditions

The Citizens Council considered that in developing its guidance NICE should not take into consideration whether or not a particular condition was self-induced (2). There were two reasons for reaching this conclusion: firstly, the Council believed it was impossible – at least in circumstances such as ischaemic heart disease – to decide whether an individual's condition was 'self-inflicted' or due to some other factor(s); secondly, the Council rejected the notion of 'deservedness' in priority setting within the NHS (2).

The board accepts that NICE and its advisory bodies should avoid denying care to patients with conditions that are, or may be, self-inflicted (in part or in whole). If, however, self-inflicted cause(s) of the condition influence the clinical or cost effectiveness of the use of an intervention, it may be appropriate to take this into account.

Principle 10

NICE and its advisory bodies should avoid denying care to patients with conditions that are, or may be, self-inflicted (in part or in whole). If, however, self-inflicted cause(s) of the condition influence the clinical or cost effectiveness of the use of an intervention, it may be appropriate to take this into account.

5.6 Patient choice

The Citizens Council emphasised in its first report (2) the importance of respecting individuals' systems of values, as well as their cultural attitudes and religious views. The Council also drew attention to the importance of individual choice. However, it recognised that individual choice would sometimes be necessarily limited in the interests of the population as a whole.

The Institute endorses the Council's sentiments, which reach to the heart of the requirement to respect an individual's autonomy. Nevertheless, while respect for autonomy and individual choice are important for the NHS and its users, this should not have the consequence of disadvantaging NHS users as a whole by having an unacceptable opportunity cost or promoting the use of interventions that are clinically and/or cost ineffective.

Principle 11

Although respect for autonomy, and individual choice, are important for the NHS and its users, they should not have the consequence of promoting the use of interventions that are not clinically and/or cost effective.

5.7 Responding to criticism

The Institute's processes both allow and encourage the involvement of consultees and stakeholders (section 3.3). The board considers it to be incumbent on the Institute and its advisory bodies to respond, objectively, to the comments of stakeholders and consultees and, where appropriate, to change their views.

The board is aware, however, that there may be occasions when attempts are made (directly or indirectly) to influence the decisions of its advisory bodies that are not in the broad public interest. The board requires the Institute, and members of its advisory bodies, to ignore such attempts.

Principle 12

It is incumbent on the Institute and its advisory bodies to respond appropriately to the comments of stakeholders and consultees and, where necessary, to amend the guidance.

The board is aware, however, that there may be occasions when attempts are made (directly or indirectly) to influence the decisions of its advisory bodies that are not in the broad public interest. The board requires the Institute, and members of its advisory bodies, to resist such pressures.

6 Social value judgements – conditions

Just as there are social value judgements relating to users of the NHS (section 5) that the Institute and its advisory bodies should take into account when developing NICE guidance, so there are social value judgements relating to the condition.

6.1 Quality and quantity of life

As discussed previously (section 4), the board considers that NICE guidance should incorporate wherever possible in its cost–utility analyses the influence of particular interventions on both the quality and quantity of life. It is for this reason that the board's preferred metric for health gain is the QALY.

The Institute is conscious, however, that incremental cost-effectiveness ratios may not adequately incorporate important features. In particular, QALYs may undervalue or even ignore the effect of a particular disease on pretreatment health status or the prognosis of the condition. Equally, conventional cost–utility analysis, and its emphasis on 'capacity to benefit', may lead to treatments not being recommended for subcategories of patients or conditions for which they would be clinically effective and cost-effective treatments.

6.2 Communicable diseases

As recognised by the Citizens Council (1), treatments used to control, or cure, communicable diseases may have benefits that extend far beyond those affected by the condition. It may not always be practical to incorporate these into the cost–utility analysis of the particular intervention and where this applies, or if the estimates are subject to substantial uncertainties, the board suggests that the Institute and its advisory bodies consider taking a more generous view of cost effectiveness.

6.3 Conditions associated with stigma

Some conditions, especially mental illness and sexually transmitted diseases, are associated with a stigma. The Citizens Council considered that some priority should be given for the treatment of such disorders (2).

Some diseases with attached stigmas have, in the past, been given a lower priority than they deserved within the NHS. There may therefore be a case for such interventions, particularly in the fields of mental health and sexually transmitted diseases, to be given some priority. The board, however, urges the Institute and its advisory bodies to be sparing in the use of special considerations and to do so only if there is reasonable evidence that those who suffer have additional psychological burdens, due to the associated stigma, that have not been taken into account in the cost–utility analyses.

Principle 13

Priority for patients with conditions associated with social stigma should only be considered if the additional psychological burdens have not been adequately taken into account in the cost–utility analyses.

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Glossary of terms

Academic in confidence See 'In confidence material' (25).

Advisory bodies The Institute's advisory bodies involved in the construction of NICE guidance comprise the two technology appraisal committees, the Interventional Procedures Advisory Committee, the subject-specific guideline development groups and their guideline review groups.

Technology appraisal committee A standing advisory committee of the Institute that develops guidance, for the NHS, on the clinical and cost effectiveness of individual (or groups of related) health technologies. Its members are drawn from the NHS, patient/carer organisations, relevant academic disciplines and the pharmaceutical and medical devices industries.

Beneficence Beneficence refers to the obligation to benefit individuals (see section 2.1.3).

Bioethics The ethics of medical and biological research and practice.

Capacity to benefit In health economics this refers to the potential increase in the health of an individual or group (see also 'Health gain') that might be achieved through the use of health services (54).

Carer (caregiver) Someone (usually a relative or close friend), other than a health professional, involved in caring for a person with a medical condition.

Citizens Council The Citizens Council is a formal committee of the Institute consisting of 30 members drawn from the population of England and Wales. The council exists to help develop the broad social values that NICE should adopt in preparing its guidance (see section 1.6.2).

Clinical efficacy The extent to which an intervention is active when studied under controlled research conditions (25).

Clinical effectiveness The extent to which an intervention produces an overall health benefit in routine practice (25).

Clinical guideline Systematically developed statements to assist practitioner and patient decisions about appropriate healthcare for specific clinical circumstances.

Clinician A healthcare professional providing healthcare. Examples include doctors, nurses, pharmacists, paramedics and physiotherapists.

Commercial in confidence See 'In confidence material'.

Communicable diseases An infectious disease due to an infectious agent (such as a bacterium, virus or parasitic worm) that arises through its transmission from another infected person, animal or reservoir (swamps, contaminated needles etc.) to a susceptible host (55).

Consultees Stakeholders within the technology appraisals programme. They include relevant healthcare professionals, patients or patient advocacy groups, and representatives of the particular manufactured technology.

Cost-effectiveness analysis An economic study in which the consequences of different interventions are measured using a single outcome, usually in 'natural' units (for example, life years gained, deaths avoided, heart attacks avoided, cases detected). Alternative interventions for the same condition are then compared in terms of cost per unit of effectiveness (54).

Cost-effectiveness model An explicit mathematical framework that is used to represent clinical decision problems and incorporate evidence from a variety of sources so that the costs and health outcomes can be estimated (25).

Cost–utility analysis A form of cost-effectiveness analysis in which the units of effectiveness are expressed as quality-adjusted life years (QALYs) (54).

Declarations of interests The requirement for members of the NICE board, the staff, and the members of its advisory groups to indicate their financial interests any technology, or business, under consideration.

Directions Legally binding instructions to the Institute (or other NHS bodies), from the Secretary of State, on the conduct of its affairs.

Disability-adjusted life year (DALY) A measure of the burden of disability-causing disease and injury (54).

Distributive justice A term used by philosophers to indicate how resources might be most appropriately be distributed in a society. For healthcare, it is concerned with the provision of services in a fair, equitable and appropriate manner in the light of what is due, or owed, to people (section 2.1.4).

Effectiveness See 'Clinical effectiveness'.

Efficacy See 'Clinical efficacy'.

Efficiency In healthcare efficiency, at its simplest level, involves using the available resources in a manner that maximises the health of the population as a whole (37), but more complicated accounts are available (54).

Egalitarianism An egalitarian considers that should get the same, or be treated the same, or be treated as equals. Egalitarian doctrines tend to express the idea that all human persons are equal in fundamental worth or moral status.

Equity For NICE, equity refers to fairness in the ways in which the costs and benefits of available care are distributed among all those who use the NHS (37) but more extensive accounts are available (54).

Establishment orders (NICE's) The legal instruments establishing the Institute, authorising its legal powers, and indicating the arrangements for its governance.

Evidence Information on which a decision or guidance is based. Evidence is obtained from a range of sources including randomised controlled trials, observational studies, expert opinion (of clinical professionals and/or patients) (25).

Guideline development group A multidisciplinary group, usually involving 12 to 15 people, with responsibility for developing a clinical guideline for NICE.

Grey literature Reports that are unpublished or have limited distribution, and are not included in the common bibliographic retrieval systems.

Health economics The application of economic theory to phenomena and problems associated with health (54).

Health-related quality of life A combination of an individual's physical, mental and social well-being; not merely the absence of disease (25).

Health technology Any method used by those working in health services to promote health, prevent and treat disease and improve rehabilitation and long-term care. Technologies in this context are not confined to new drugs or sophisticated equipment, but include surgical procedures, devices and other forms of therapeutic intervention such as physiotherapy and psychology.

In confidence material Information (for example, the findings of a research project) defined as 'confidential' because its public disclosure could have an impact on the commercial interests of a particular company or the academic interests of a research or professional organisation (25).

Incremental cost-effectiveness ratio (ICER) The ratio of the difference between the costs of two alternatives and the difference between their effectiveness (54).

Interventional Procedures Advisory Committee A standing committee of the Institute's board responsible for advising on the safety and efficacy of interventional procedures.

NICE guidance NICE guidance includes technology appraisals guidance, clinical guidelines, and interventional procedures guidance. It will also, in the future, include public health guidance.

Non-maleficence This asserts an obligation not to inflict either physical or psychological damage (section 2.1.2).

Opportunity cost The opportunity cost of investing in a healthcare intervention is the other healthcare programmes that are displaced by its introduction. This may be best measured by the health benefits that could have been achieved had the money been spent on the next best alternative healthcare intervention.

Outcome Measure of the possible results that may stem from exposure to a preventive or therapeutic intervention. Outcome measures may be intermediate endpoints or they can be final endpoints (25).

Public health The science and art of preventing disease, prolonging life and promoting health through organised efforts of society.

Quality-adjusted life year (QALY) A generic measure of health-related quality of life that takes into account both the quantity and the quality of life generated by interventions.

Quality of life See 'Health-related quality of life'.

Social value judgement An ethical opinion made either implicitly or explicitly that a particular course of action, institutional arrangement or method of analysis ought to be implemented, or is itself good (54).

Stakeholder Those with an interest in the use of a technology under appraisal or a guideline under development. Stakeholders include manufacturers, sponsors, healthcare professionals, and patient and carer groups (25).

Synthesis of evidence A generic term to describe methods used for summarising (comparing and contrasting) evidence into a clinically meaningful conclusion in order to answer a defined clinical question. This can include systematic review (with or without meta-analysis), and qualitative and narrative summaries (25).

Systematic review Research that summarises the evidence on a clearly formulated question according to a predefined protocol using systematic and explicit methods to identify, select and appraise relevant studies, and to extract, collate and report their findings. It may or may not use statistical meta-analysis (25).

Technology See 'Health technology'.

Technology appraisals Recommendations on the use of new and existing medicines and other treatments within the NHS in England and Wales, such as: medicines (for example, drugs), medical devices (for example, hearing aids and inhalers), diagnostic techniques (tests used to identify diseases), surgical procedures (for example, repair of hernias), and health promotion activities (for example, patient education models for diabetes) (25).

Technology assessment The process of evaluating the clinical, economic and other evidence relating to the use of a technology so that guidance on its most efficient use can be formulated (25).

Utilitarianism This is an ethical doctrine which specifies 'utility' as the principal good characteristic of society: what humankind as a whole ought to maximise (54).

Utility Utility is number assigned to entities (usually benefits or things presumed to be the objects of people's preferences) according to a rule. This enables the entities to be quantified and ranked according to preference, desirability or choice (54).

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)

PHARMAC AND THE FUNDING OF HIGH COST PHARMACEUTICALS

Raanan Gillon, Emeritus Professor of Medical Ethics, Imperial College London

PHARMAC has asked for responses to the following questions:

a. What are the main economic/social justice/ethical theories relevant to how decisions on funding "high cost" pharmaceuticals could be made?
b. What if any justification is there for assessing High Cost Pharmaceuticals [HCPs] differently from other pharmaceuticals considered for public subsidisation?
c. What might be the downsides of valuing HCPs differently?
d. Could cost utility analysis be used more effectively when considering HCPs ? If so, how?
e. What if any changes do you recommend PHARMAC make to its current decision making process for HCPs ?
f. What role should rule of rescue play in assessing HCPs ?
g. What are the arguments for and against paying a higher price (per QALY gained for example) for pharmaceuticals for those who are worse off clinically with poor quality-adjusted life expectancy, but of arguably greater need (for example the terminally ill)?
h. Are there any general comments that you wish to make ?

In this paper I first respond to question a) and within that section briefly respond to question g). I then respond to questions b) c) and f). I end with a response to question e). I do not respond to question d) as it is not within my area of expertise to do so; and I make some general comments (question h) intermittently within the paper, and in the last paragraph.

a) What are the main economic/social justice/ethical theories relevant to how decisions on funding "high cost" pharmaceuticals could be made?

There is a wide variety of substantive ethical theories and principles of distributive justice with no one theory commanding even wide acceptance let alone universal acceptance. Ruefully noting this lack of agreement in 1985, I outlined Aristotle's still widely agreed *formal* principle of justice and five potentially mutually inconsistent *substantive* theories of justice that nonetheless could conform to the Aristotelian principle (that equals should be treated equally and unequals unequally in proportion to the relevant inequalities). These were libertarian theories, utilitarian theories, Marxist theories, Rawls's theory and desert-based theories[11]. Twenty years later Lamont, in the online Stanford Encyclopedia of Philosophy[2] discusses a wider range of substantive principles of distributive justice, all potentially consistent with the Aristotelian formal principle but mutually inconsistent. The theories described by Lamont are those based on:

strict egalitarianism (in which everyone has to have the same level of material goods and services);

the difference principle (based on Rawls's theory of justice in which everyone first has an equal claim to equal basic rights and liberties and secondly social and economic inequalities are considered just only if they are attached to positions and offices open to all under conditions of fair equality of opportunity and are such as to bring the greatest benefit to the least advantaged. An important application of Rawls's theory of justice to health care is Norman Daniels's theory of 'Just health care' in which ill health and disability are seen as impairing people's fair equality of opportunity. An important variant on Rawls's theory of justice is Nobel Prize-winning economist Amartya Sen's theory of justice in which equality of capability to achieve what one values is the proper objective of justice);

resource egalitarianism (based on the notion that justice requires equality of resources but that people's use of their resources can be justly various and that those who for example work

harder to build up their resources do not owe subsidies to those who choose to work less hard and thus obtain less income. Resource egalitarians such as Ronald Dworkin agree that compensation for natural disadvantages over which people have no control would be required by their theories of justice)- this group and the last group of principles of justice are sometimes called '**egalitarian-liberal' theories of justice**;

welfare maximisation (based on the utilitarian basic claim that maximising people's welfare is the fundamental moral concern and that just distribution of scarce resources is whatever distribution maximises welfare- various types of welfare maximisation have been described including Bentham's 'greatest good for the greatest number' and contemporary utilitarian concern to maximise people's preference satisfaction)- **utilitarian justice**;

people's deserts (based on the notion that scarce resources should be distributed in proportion to what people deserve- typically because of their virtues and vices (Aristotle) or their 'toil' (John Locke) or in modern variants, their merits and demerits, their work effort and costs, their productivity and their retrospective and prospective contribution to society)- **desert-based justice**;

libertarianism (In Robert Nozik's libertarian theory of justice it is not outcome that is morally relevant but the moral nature of individual transactions; if a person justly acquires a holding he or she is entitled to it and when a person justly transfers a holding to which he or she is entitled then the acquirer is entitled to that holding, and no one is entitled to holdings except by such just acquisitions, and overall distributive justice is achieved by exclusion of all transactions that are not based on such just entitlements and compensation for those that have been transacted contrary to just entitlements)- **libertarian justice**;

feminism (Lamont points out that there is a wide range of feminist perspectives on justice but concentrates on liberal feminist perspectives that claim equal rights for women as for men, and a concern to remedy injustices against women that stem from liberal reluctance to allow the state to interfere in the 'private' sphere in which women are in practice unjustly treated. In addition feminist care and relationship perspectives such as those associated with the work of Carol Gilligan may be seen as supplanting impersonal principles such as the principle of justice)- **feminist approaches to justice**.

To these broad categories of theories of distributive justice we should add:

Kantian theories (in which justice is determined by application of Kant's 'categorical imperative' according to which justice is obtained when people are treated never merely as means to an end but always as 'ends-in-themselves'- as autonomous beings; when moral agents base their actions on maxims or rules of thumb that can be legitimately 'universalised' so that anyone in a similar situation could morally acceptably act on the same maxim; and when moral agents act on the basis that they are legislating for a kingdom of ends-in-themselves, or autonomous beings)- **Kantian justice**.

Marxist theories (in which justice is most pithily summarised in the slogan 'to each according to his need, from each according to his ability') **Marxist justice**;

Religious theories (in which, typically, differential concern favouring the poor, the suffering and those in need is emphasised)- various **religious theories of justice**

Virtue theories (in which justice is the result of and determined by the attitudes and actions of virtuous people[3])- **virtue based theories of justice**

Pluralist theories of justice (in which a variety of potentially conflicting moral concerns are built into theories of justice)

Anti-racism theories of justice (in which, as with many feminist theories, emphasis is placed on remedying systematic injustices to particular groups, in this case those experienced by oppressed racial groups)

Environmental justice (whose emphasis is on justice based on environmental sustainability)

Rights based justice (in which justice, increasingly including distributive justice, is based on people's perceived human rights, and especially their human rights as enshrined in international and national conventions, declarations and laws. This approach to justice is, unsurprisingly, especially favoured in legal (as distinct from philosophical) perspectives on justice, and of course in poorer parts of the world, where so called positive rights, requiring assistance -including aid- from others, are often seen as more important that negative rights which simply require others to desist from actions (such as unjust oppression, torture, imprisonment, restriction of liberties such as political association and so on). Aspects of human rights conceptions of justice have increasingly been democratically incorporated- typically through Human Rights Acts- into national laws including those of New Zealand.

No single theory of justice commands widespread acceptance

The huge range of substantive theories and principles of justice, briefly indicated above, demonstrates the fact that no one theory commands anywhere near universal acceptance. I believe that it also demonstrates the range of morally relevant concerns that an adequate substantive theory of justice would have to accommodate. In a paper[4] for the UK Health Equity Network I outlined criteria, broadly reflecting a range of competing theories of justice, that would be plausibly necessary for a substantive theory of distributive justice to command widespread acceptance. The paper reiterates that while an underlying acceptance that justice requires equality in some sense or another is common to all theories of justice, as Aristotle pointed out so long ago equality is not necessarily just or fair. His underlying formal requirement for justice, that equals be treated equally and unequals be treated unequally in proportion to the relevant inequality (what contemporary health economists sometimes refer to as horizontal and vertical equity) remains widely accepted. However philosophers, politicians, theologians, amongst many other groups, have been arguing ever since about what are the relevant inequalities that justify treating people unequally (vertical justice), as well as what are the relevant equalities that justify treating people equally (horizontal justice). Equality remains the central concern, but as Amartya Sen emphasises, the crucial issue is 'equality of *what?'* [5].

Equal health care resources for equal health care need

In health care contexts variations in levels of **health care need** are widely accepted to be morally relevant inequalities such that the greater the need the greater the presumption of a moral obligation to try to meet that need[6]. This of course leads to morally required ***in***equality of treatment. Unequal treatment in proportion to need is built in to PHARMAC's existing overarching objective (1.1) and health needs are referred to in three of the nine decision criteria in PHARMAC's operating policies and procedures document (2.2 a,b and e). A moral obligation to benefit people differentially (ie unequally) in proportion to their need is found in a variety of moral theories including those of the major religions and of Marxism. Doyal and Gough[7] are contemporary exponents of a needs based system for allocation of health care resources.

Unfortunately for those who would like a relatively simple theory of distributive justice, (a) there are problems associated with the very concept of distribution of resources in proportion to health care need and (b) various other criteria morally relevant to just distribution of scarce resources can conflict with distribution in proportion to people's need for them.

Problems with the criterion of distribution in proportion to health care need

These can be summarised as problems with the concepts of need in general and of health care need in particular; the potential incommensurability of different sorts of need, and of different sorts of health care need; the problems of unmeetable needs; and problems of needs that while they are meetable have either a very low probability of being met, or else can only be met at very high cost.

So far as the concept of need goes, I shall simply assert that in a highly contended arena my own working account of a need is **that without which one is harmed**[8]. A health need is that without which one's health is harmed, and a health care need is an element of health care without which one's health is harmed.

Can different sorts of needs be compared?

The incommensurablility , or non-comparability, of needs is a subclass of the general problem of incommensurability of benefits and harms. How can one say that relieving severe itching is better or worse than gaining an extra day of life, let alone how much better or worse? Like chalk and cheese we simply can't compare them- or can we? Suffice it summarily to say here that since people are in fact able to make choices between incommensurables, and indeed to give differential 'weights' to incommensurable alternatives this problem is in practice surmountable. The use of QALYs (quality adjusted life years) in the context of health care provision is one way of surmounting it. The use of QALYs is open to a variety of criticisms[9],[10] to some of which I shall return below, but in the context of health care need the main problem is that while they may provide a way of overcoming the incommensurabilty problem they afford no basis for distinguishing between QALYs gained in the context of meeting needs, and QALYs gained in the absence of need. A QALY gained as a result of my going to the South of France is equivalent to a QALY gained in relieving my pain or zapping my cancer. Even within the context of QALYs gained from health care the QALY gained by the insomniac is equivalent to the QALY gained by the patient in heart failure.

Meetable and unmeetable needs

Even if needs are graded in terms of severity of ill health/disability and made – at least de facto- commensurable (for example by measuring a QALY deficit from normal species functioning seen as equivalent to 'adequate health') not all needs can be met, and this would be true even were there to be unlimited resources (and of course there never are or will be unlimited resources). Thus as already noted[6] a person dying from a disease for which there is no known cure has a need for such a cure- but that need cannot be met. There is no point in, and in the context of scarce resources no moral justification for, providing treatments that cannot provide benefit. On the other hand, (and to respond briefly to PHARMAC's question g) above), people dying from incurable conditions remain in enormous health care need (primarily of cures for their fatal condition) and if this large degree of health care need would justify prioritising successful treatment, it seems reasonable to prioritise meeting their ***meetable*** health care needs even in cases when their major need for a cure for their condition can not be met. On this basis-in the context of pharmaceuticals- medications that are curative for other conditions but that are known to be ineffective for a particular patient's fatal condition should not be provided in an ineffective and often deceptive attempt to 'do something'. However medications that meet such patients' ***meetable needs,*** for example by alleviating their distressing symptoms such as pain, nausea and vomiting, itching and other unpleasant sensory experiences, psychological disturbances of various sorts, auditory and visual disturbances and indeed any other distressing symptom- should be prioritised.

As well as patients with very great needs that cannot be met there are also patients with very great needs for whom **treatments *might* meet those needs but with only a low probability**

of doing so. And there are patients with very great health care needs that are clearly meetable but only at *very high cost.* In both cases there is obviously a major moral tension between on the one hand providing beneficial treatment for those who greatly need it and on the other hand the opportunity costs to those denied beneficial health care resources that are spent instead on meeting very few people's needs at very high cost, either because the individual treatment though likely to be effective is very expensive or, in the case of low probability of successful treatment, because much resource will be wasted on providing non-beneficial (and possibly harmful) treatments to most recipients. I shall return to these issues when considering high cost pharmaceuticals and 'the rule of rescue'.

Producing sufficient, let alone maximal, benefit can conflict with meeting needs

Clearly, however, ***production of sufficient benefit*****, let alone production of maximal benefit** (the moral basis of utilitarian ethical theories) can conflict with distribution in proportion to need and an adequate theory of justice must surely have ***some*** place for a concern to produce a ***sufficiency*** of beneficial outcomes in the use of scarce resources. It would be, to say the least, highly counterintuitive for a theory of distributive justice to advocate the use of scarce resources on treatments that produced very little benefit, or had very low probabilities of producing benefit, any more than it could plausibly advocate using most of the available resources on producing great benefits for just a few recipients, even if those few had very great needs for such treatments. And, of course, built into PHARMAC's existing policy is exactly such an outcome concern to produce sufficient benefit per unit of resource used, notably its concern to limit the cost per unit of beneficial outcome (measured in QALYs) that it is normally prepared to pay for new pharmaceuticals. It is worth noting in this context that PHARMAC's existing policy is (implicitly) oriented to producing ***sufficient*** rather than ***maximal*** benefit and that it is explicitly concerned with benefit in relation to cost; cost-effectiveness is explicitly one of its criteria (eg OPP 2.2 (e) and 4.4), when comparing comparable outcomes: Indeed, cost utility analysis (CUA) using QALYs and direct financial costs for comparing different sorts of health interventions and outcomes (A Prescription for Pharmacoeconomic Analysis [PPA]- 1,3), is in practice probably its most important allocation criterion. Criteria for sufficiency of benefit are not specified in the current OPP and PPA, but pharmaceutical interventions that cost $NZ10, 000 or less per QALY gained are likely to be accepted as producing sufficient benefit (see eg PHARMAC memo of Oct 2003 on High Cost Pharmaceuticals, p2) whereas those costing more than $NZ10, 000 per QALY gained require special consideration. It is however explicitly stated that maximising beneficial outcomes as indicated by CUA is sometimes to be over-ridden by other criteria including avoidance of discrimination against the elderly and an element of preference for life-saving interventions over interventions that improve quality of life but do not extend life expectancy (PPA pp9-10). I shall return to this when considering the issue of 'the rule of rescue'.

Conceptually linked to beneficial outcome criteria are the **efficiency** criterion (avoidance of waste, or non-beneficial use, of scarce resources), and the **opportunity cost** criterion (consideration and justification of benefits that would be foregone by any proposed use of scarce resources). Both of these criteria may also conflict with allocation in proportion to need but (as a non-economist) I take it that avoidance of waste is an integral part of both cost effectiveness analysis and cost utility analysis, and that reduction of opportunity cost is its underlying moral justification.

Respect for people's autonomy can conflict with meeting needs

Respect for people's autonomy is another morally relevant criterion to be included in an adequate substantive theory of distributive justice but it too may conflict with treating people equally in proportion to their needs, and it may also conflict with maximising benefit. Again PHARMAC's operational functioning at least implicitly accepts the relevance of this criterion. Thus no one is forced to submit medications to PHARMAC, or to prescribe

medications authorised by PHARMAC, or to use medications that have been prescribed for them (even if such coercion were to meet needs and provide benefit or even maximise benefit). And the autonomy of 'the people' as represented by their democratically elected Government is, arguably, respected insofar as the budget and priorities for its expenditure is accepted in PHARMAC's objectives and operational functioning.

Respect for autonomy does not mean accepting 'I want it so do it'

In this context it is important to note that, at least according to standard accounts, respect for autonomy does **not** require that one does what another person autonomously requests or requires one to do- 'I want it so do it'- it simply requires that one does not interfere with the person's own autonomy (literally self-rule, probably better briefly described as deliberated, or thought out, self rule)- always with the qualification that such respect has to be compatible with equal respect for the autonomy of all potentially affected. Thus the corresponding right is the negative right of not being prevented from acting autonomously, insofar as such 'deliberated self-rule' is compatible with others' autonomy. Of course if one does do what another autonomously requests or requires one to do then that is entirely ***consistent with*** the principle of respect for autonomy but it is not ***required*** by the principle. Thus if a doctor insisted on administering to a patient some medication or other treatment despite the person's autonomous refusal that would infringe the person's autonomy and would thus infringe the principle of respect for autonomy (even if the medication or other treatment were unquestionably needed and beneficial). But if a doctor refused to comply with a patient's request or demand for a particular medication, or other treatment, that would ***not*** infringe that patient's autonomy and would thus not infringe the principle of respect for autonomy (even though giving the requested medication or treatment would be entirely consistent with respecting the person's autonomy and thus with the principle- and even if in some cases refusal might be immoral on other grounds- for example if it constituted failure of the doctor's duty of care).Similarly if PHARMAC refuses to authorise a medication for Government subsidy despite the autonomous requests/demands of pharmaceutical manufacturers, doctors, patients or patient advocates, such refusal does not infringe the principle of respect for autonomy even though authorisation of the medication would be entirely consistent with the principle and in some cases may be required by some other moral concern, especially provision of health benefit.

In relation to Government matters are different, for Government, on behalf of the people it democratically represents, has created PHARMAC as its/their agent equitably to manage government expenditure each year of a specified amount of money on subsidising pharmaceuticals: the sum of money and the eligible population and the type of health care expenditure (on pharmaceuticals) are specified by Government as are several criteria for equitable distribution including 'the best health outcomes' for 'people in need' (OPP 1.1) within the confines of NZ law (1.2.3 and 1.6.3), in accordance with the Treaty of Waitangi (1.6), which explicitly requires responsiveness to 'the particular characteristics, special needs and cultural values of Maori communities' (1.6.2) insofar as this is consistent with avoidance of racial discrimination and the Human Rights Act 1993 (1.6.3). The Government also reserves the right to require PHARMAC to implement the Government's 'priorities for health funding' (2.2 (h)). In respecting these obligations PHARMAC can, perhaps optimistically, be seen to be respecting the autonomy of the people of New Zealand collectively, to the extent that democratically elected governments represent such collective autonomy (and ignoring here the manifold problems of 'democratic deficit'). For PHARMAC to spend more than its allocated budget, or to spend it on unauthorised expenditure (for example on health care for people deemed by Government to be ineligible) would be to infringe the autonomy of those providing the funding as represented by their democratically elected Government. Thus this is a very different case from PHARMAC not spending its budget on treatments requested or demanded by pharmaceutical companies, doctors or patients and patient groups, where refusal would not infringe the autonomy of these stakeholders, even though expenditure in

accordance with such requests or demands would be consistent with respect for their autonomy.

Prioritising particular groups and relationships can conflict with distribution in proportion to need

Another at least plausible, yet highly contentious, criterion for an acceptable theory of distributive justice is **prioritisation of the needs of certain groups on the basis of certain sorts of relationships** and their concomitant commitments. This criterion too may conflict with allocation in proportion to need.

Doctors prioritising the interests of their patients

In particular, once a patient is in a professional relationship with a doctor, or other health care worker, that relationship establishes a psychological bond and an element of consequent commitment such that the doctor or other health care worker naturally gives priority to the interests of that patient over the interests of other people with whom he or she does not have a professional relationship. This psychological bond is strengthened by an explicit professional moral commitment of doctors and other health care workers to protect the health interests of **their** patients. This sort of special commitment by individual doctors and other health care workers to the health interests of 'their' patients can clearly conflict with distribution of resources according to need. But once again it would be an intuitively implausible substantive theory of distributive justice that did not accommodate ***some*** degree of such differential concern. In the context of distribution of pharmaceuticals this differential concern is already in practice somewhat uneasily acknowledged, in that doctors are not merely permitted but expected to give priority to their patients. Within their groups of patients doctors are expected to prioritise in relation to medical need, but they are nonetheless expected to give priority to the needs of their own patients over the medical needs- even the greater needs- of others. This expectation has fuzzy borders for in emergencies doctors are also expected to prioritise the medical needs of strangers with whom they have had no previous professional relationship, even though this may be at the expense of the lesser needs of their own patients. Furthermore within national health services doctors are increasingly expected to limit their prescription of medications, especially expensive medications, according to protocols approved by government-controlled agencies. While patient safety is an important objective of such protocols, rationing in pursuit of distributive justice is often another and increasingly doctors are expected to follow such protocols even though they may conflict with the best interests of some of **their** patients.

Nations prioritising the interests of their nationals and residents

This uneasy prioritisation of the needs of their own patients while acknowledging some, but undoubtedly lesser, responsibility to meet the needs of others is reflected at a national level (and at least in the UK at regional levels too). Thus as the PHARMAC's OPP make clear (eg OPP1.1 and 2.2 (a)), it is the NZ government that decides eligibility for subsidised pharmaceuticals, and it is reasonable to presume that the bulk of such subsidy is provided for NZ nationals (and eligible Pacific Islanders) and a small part for others resident in NZ. Other people, in other parts of the world, are for the most part excluded from such subsidy, irrespective of their medical need for pharmaceuticals, though doubtless some relatively small provision is made within the NZ overseas aid budget towards meeting such needs.

However the fact that special relationships and their associated commitments are in practice an established component of systems for allocating scarce medical resources does not entail that they are morally justified. Suffice it to assert that even utilitarian justice arguments based on traditional welfare maximisation and/or preference satisfaction maximisation are likely to support such differential allocations, as are justice arguments based on respect for autonomy.

People are likely to prefer, even after autonomous deliberation, that doctors have a differential concern for their own patients (as distinct for example from requiring them to provide their services on the basis of a strict needs related egalitarianism). In national health services funded through taxation voters are likely to prefer that the bulk of the funding is distributed to their own population rather than globally on the basis of a strict needs-related egalitarianism. Theories of justice that prioritise the importance of caring relationships (for example some feminist theories) can be expected to acknowledge the importance of such relationships in the context of allocation of scarce medical resources. In all such theories the role of special relationships in distributive justice will have to be tempered by other morally relevant criteria such as those mentioned above, but in all such theories some priority is likely to be given to those in special relationships. One important practical implication of accepting the moral importance of special relationships is that once a patient is receiving subsidy for a beneficial medication, continuation of that subsidy for that patient should be prioritised, even when subsequent decisions rescind authorisation of subsidy for the medication. This does not entail continuation of subsidy for a medication after it ceases to be beneficial to a particular patient, though conditions for subsidy (eg 'so long as the medication continues to provide [objectively determinable?] health benefit') should be made clear to doctors and patients (or their representatives) before the medication is started.

Elimination and prevention of morally unacceptable use and distribution of scarce resources can also conflict with distribution in proportion to need

Another criterion that would be relevant to an adequate substantive principle of distributive justice is the elimination and prevention of morally unacceptable use and distribution of scarce resources. Prevention and detection of fraud and other forms of cheating, for example, or elimination of geographical inequities of access, or prevention and detection of morally unacceptable racial or gender or other illicit discrimination, or provision and implementation of public scrutiny and accountability mechanisms can all thus be relevant to fair distribution of scarce resources but, given a fixed quantity of resource they can all, perhaps paradoxically, conflict with beneficial use of those resources in proportion to need and reduce the welfare outcome of the available resource.

I do not see any reference to this issue in the PHARMAC literature and it might be worth considering it. One approach might be to consider any such expenditure that paid for itself as always worth funding, and to set a normal limit (eg not more than x per cent of total budget- where the size of x would be a political judgment) on the amount of any other such expenditure. But opposition can be expected from those who strongly prioritise elimination of unjust distributive factors- some may well adhere to the old adage, 'let justice be done though the heavens fall'- ie discovery and elimination of unjust factors affecting allocation of PHARMAC's resources would for them take absolute precedence over other expenditure, including expenditure on health care.

All these criteria- meeting needs, producing maximal or sufficient benefit, respecting autonomy, giving preferential weight to certain 'relationship obligations' such as the health needs of a doctor's patients or a regional or ethnic group, or a country's nationals, and elimination and prevention of clearly unjust methods of allocation- are potentially relevant to a widely and morally acceptable substantive theory of justice, and no such theory has been widely accepted nor to the best of my knowledge exists[11]. Thus from PHARMAC's point of view it seems far preferable to acknowledge the wide range of potentially conflicting moral criteria, the lack of an agreed unifying substantive theory of distributive justice and to seek to specify the criteria that seem to it relevant in particular cases where these conflict, and to make explicit its approach to resolving such conflicts when they occur- about which I shall say more towards the end of this paper.

PHARMAC's Questions b) c) and f):
b) What if any justification is there for assessing High Cost Pharmaceuticals [HCPs] differently from other pharmaceuticals considered for public subsidisation?
c) What might be the downsides of valuing HCPs differently?
f) What role should rule of rescue play in assessing HCPs ?

In response to b) I shall assume that the bulk of pharmaceutical assessments done by PHARMAC are based on the notion that the pharmaceutical to be assessed has been licensed as effective in meeting some medical need. A cost utility assessment is carried out and if the pharmaceutical is calculated to cost less than c $NZ10, 000 per QALY produced, the product is subsidised. The underlying rationale for this approach can be interpreted to be that a pharmaceutical treatment that effectively meets some health care need is 'affordable' if it produces health gain of one QALY for less than $NZ10, 000, and should therefore be subsidised. It doesn't matter whether the need being met by the pharmaceutical is a minor one (the itching and soreness between two toes of a minor case of athlete's foot, for example) or a major one (the pain of a heart attack for example)- provided that meeting the need costs less than $NZ10, 000 per QALY gained the pharmaceuticals should be subsidised. This approach incorporates the criteria of meeting health care needs, doing so effectively and doing so affordably, but takes no account of the ***extent*** of the needs being met. However the need criterion for distributive justice discussed above is the prima facie obligation to try to meet a patient's health care needs ***in proportion to the extent of those needs***. According to this criterion, the greater the need, the greater is the prima facie obligation to meet it. Thus a pharmaceutical that fails to meet the affordability criterion of $NZ10, 000 per QALY may nonetheless be capable of effectively meeting a severe health care need, and it is this fact that may justify 'assessing HCPs differently from other pharmaceuticals considered for public subsidisation' (question b) above). The HCP is not initially assessed differently, for all pharmaceuticals go through the same initial assessment by PHARMAC; but pharmaceuticals that would cost significantly more than $NZ10, 000 per QALY gained require additional assessment if they effectively meet severe health care needs. The 'downside' of such additional assessment is sub-maximal health gain per unit of resource and sub-optimal reduction of opportunity cost- but as I have argued above, sufficient health gain is only one of many potentially competing criteria within a potentially widely acceptable theory of distributive justice; with effective treatment of health care need in proportion to the severity of that need being widely regarded as more morally important than maximising benefit.
Severity of health care need can itself be assessed along the two dimensions of impairment of quality of life and impairment of length of life, and advocates of cost per QALY assessments may well argue that no further assessment is justified, for QALYs are precisely designed to combine quality and length of life assessments. Unfortunately such responses encounter two major obstacles. The first is that cost per QALY gained assessments do not incorporate comparative assessments of prior health care needs- a QALY gained in treatment of athlete's foot is as valuable as a QALY gained in treating the pain of a heart attack. The need criterion requires such comparative assessments for it gives priority to treating greater needs over treating lesser needs. The second obstacle is that QALY assessments combine quality of life and length of life assessments, giving no priority to one or the other. But there are widespread human tendencies to see these assessments as fundamentally different and incommensurable- and to give moral priority to attempts to meet life- threatening needs, presumably on the grounds that it is more morally important to try to meet life threatening needs than to try to meet non-life threatening needs.

The priority of saving life- not absolute but not just a matter of QALY assessment either

This widespread human tendency to prioritise the saving of life over other beneficial activities for others is especially directed at saving lives that are identified and in some sense 'near'. The tendency has been dubbed 'the rule of rescue'[12] and summarised by Hadorn as - 'the powerful human proclivity to rescue endangered life'[13] -though probably 'the powerful

human proclivity to ***wish*** to rescue ***near and identified and immediately*** endangered lives' would be nearer the mark since even casual observation indicates an equally powerful human proclivity to ***wish*** to rescue rather than to rescue, and to ignore endangered lives that are not in any sense 'near', that are unidentified, and that are not in immediate danger. But even the thus qualified human tendency to wish to rescue endangered lives is of enormous moral importance in promoting the moral norm of beneficence to others in great need. However, when resources are limited, as they usually are, very expensive beneficence to some conflicts with (some of the) other moral obligations of justice identified above, and in particular with the opportunity cost to those people whose needs will not be met as a result of expenditure on very expensive rescues of those in great need. This will be the case even if we consider only life saving needs, for it hardly needs saying that within a fixed budget more lives can be saved with cheaper life saving means than with very expensive life saving means! But far more morally difficult is to try to 'weigh up' the benefits of life saving activities against the opportunity cost for people whose health care needs are not for life saving but for improvement in their quality of life.

Two polar responses are encountered, neither ultimately widely acceptable. The first is to accept that saving life must always take priority over other health care interventions that do not prolong life. The other is simply to deny that there is any morally justifiable distinction to be made- cost per unit of benefit is the only morally important criterion according to this response, with maximisation of benefit per unit of resource the guiding moral principle. Quality adjusted life years integrate the benefit of extension of life with the benefit of a healthy quality of life so maximisation of QALYs gained per unit of resource expended provides a method for maximising benefit. To start with this second response, which is essentially a simple version of the utilitarian moral criterion, it is open to objections both from within a more sophisticated utilitarian framework and from alternative moral perspectives. Sophisticated utilitarians can argue that failure to allocate additional value to life saving activities fails to acknowledge that life is the necessary condition for any sort of pleasure, satisfaction (including preference satisfaction), happiness, eudaimonia or any other sort of beneficial quality of life. Since maximisation of one or other of these is the utilitarian objective, additional value must, at least prima facie, be attributed to life prolonging activities as compared with activities that merely improve quality of life without extending it. This response acknowledges that prolonging lives of poor quality ***may*** produce less benefit than improving the quality rather than the length of people's lives, but emphasises that in general prolonging life is likely to take priority over merely improving quality. Moreover, since there is widespread social ***desire*** for attempts to be made to rescue immediately endangered lives, again a sophisticated utilitarian can argue that priority should be given to life saving activities over those that merely improve quality of life.

Non-utilitarian perspectives are likely to emphasise the intrinsic- and for some absolute- moral importance of saving life. Judaism and Islam count the saving of life as the supreme value[14] and while there seems less absolute an emphasis on the importance of saving life in the Christian doctrine of sanctity of life (which is more concerned with an absolute prohibition of the morally unjustified taking of life), nonetheless according to a widely seen Roman Catholic website 'ordinary means' (ie in Roman Catholic parlance morally required means) of saving life include continued provision of artificial nutrition and hydration to patients in persistent vegetative state (PVS), as in the American case of Terri Schiavo[15]- indicating a very strong emphasis on saving even hopelessly damaged lives. Such absolutist religious concerns may not be widely shared, but they surely reflect widespread acknowledgment of at least a strong moral commitment to preserve life. The ever-increasing medical capacity to preserve life, however, brings this commitment increasingly into conflict with the recommendations of cost-benefit and cost utility analyses.

Should we then simply accept the other polar response, that life-saving interventions should always be prioritised over non-life saving interventions? Consideration of PVS is for most

people (though of course not all) sufficient to demonstrate the moral implausibility of any such claim for it would entail that resources would have to be prioritised to keeping everyone alive, regardless of the quality of those people's lives and regardless of the opportunity cost of doing so. The extreme example of such a position would require patients in PVS to be kept alive by ***any*** effective life-prolonging treatment, regardless of the opportunity costs to other patients needing non-life-saving treatments. Even if we assumed that people would be allowed to refuse such life prolonging treatment in advance directives, anyone who had not refused would have to be treated with life prolonging treatment until they eventually died. These treatments would not be restricted to artificial nutrition and hydration but would include all and any potentially life-prolonging treatment, including very expensive ones. The idea that a cancer patient who went into persistent vegetative state should nonetheless continue to be treated with anti-cancer therapy, along with any other treatments that would prolong his or her life, including cardiopulmonary resuscitation, should be sufficient to illustrate the moral implausibility of accepting that life-saving interventions should always be prioritised over non-life saving interventions.

The 'rule of rescue'

An increasingly articulated variant of the obligation to save life even at very high cost is the so-called 'rule of rescue'. This has been variously described, not just as Hadorn's 'powerful human proclivity to rescue endangered human life', or my suggested variant above, but also as Richardson and McKie summarise it, as 'the urge to rescue identifiable individuals facing avoidable death, without giving too much thought to the opportunity cost of doing so'[16] Richardson and McKie also report several other accounts of 'the rule', namely: 'a perceived duty to save endangered life where possible', 'the sense of immediate duty that people feel towards those who present themselves to a health service with a serious condition', 'an ethical imperative to save individual lives even when money might be more efficiently spent to prevent deaths in the larger population', 'the powerful human proclivity to rescue a single identified endangered life, regardless of cost, at the expense of many nameless faces who will therefore be denied health care ', and Hadorn's 'fact about the human psyche that will inevitably trump the utilitarian rationality that is implicit in cost-effectiveness analysis: people cannot stand idly by when an identified person's life is visibly threatened if rescue measures are available'. Bioethicist Albert Jonsen, who originally coined the term 'rule of rescue'[17] points out in a later article that while this 'imperative to rescue endangered life' is 'undoubtedly of great moral significance'; yet he adds ' the imperative seems to grow into a compulsion, more instinctive than rational'. He recounts his puzzlement at how to resolve the impasse when 'the rational effort to evaluate the efficacy and costs, the burdens and benefits, of the panoply of medical technologies- an effort essential to just and fair allocation- encounters the straitened confines set by the rule of rescue. Even the soundest consequentialist arguments against that rule seem unable to break out of the box.'[18]

Various features may tentatively be discerned within the 'rule of rescue'. The first is its lack of clarity; though the differing formulations cluster around severity and immediacy of need, with need for life saving as a common (but not universal) feature- Haddorn[13] points out that nasty fractures can evoke the same rescue response, as indeed can any severe illness or injury, and Richardson and McKie[16] point out that sometimes enormous sums are spent to fly children with non-life threatening deformities or disfigurements from poor countries to wealthy countries for treatment.. In so far as some clarity can be extracted from the 'rule' (and it is noteworthy that no index entry for 'rule of rescue' is to be found in the latest 5-volume edition of the Encyclopedia of Bioethics), it seems to prioritise, as well as severe and immediate need, those needs about which ***others have strong moral feelings,*** generally because in some sense or another they feel ***'near' to an identified person or persons in need***.

In some contexts such moral feelings may result from special and real relationships – eg those of family or friends, or the somewhat different but often nonetheless powerful feelings noted

earlier of health care workers as advocates for 'their' patients. Sometimes the feelings may arise from mere physical propinquity- the child has fallen down my well, the fire is in my road or village, the man has collapsed in front of me, and I feel I ***ought*** to try and help, try and ***rescue***. In other cases the relationship is more 'virtual' than real, for example because a person's plight has been publicised in the media- but though the relationship may be 'virtual', the evoked feelings that the person(s) identified by the media ***ought to be rescued*** may nonetheless be real and powerful.

The rule of rescue, moral dilemmas and conflicts of moral principles

In the context of high cost pharmaceuticals these various factors increasingly often produce genuine moral dilemmas. Strong moral reasons exist for treating the person in need, especially when the need is very great as in cases of life saving need. All the normal moral justifications for treatment are present, the patients would benefit, often greatly; they or their proper proxies autonomously request the treatment; their doctors wish to provide the treatment; the treatments are legally acceptable; the population may well be clamouring for the life-saving treatment to be provided. On the other hand provision of the very expensive but life-saving treatment in the context of a fixed budget can produce unfair opportunity costs for other patients in need, and be especially unfair to those whose needs though major are for other than life-saving treatments. Moreover provision of the high cost pharmaceutical will by hypothesis produce less overall health gain than alternative uses of the resources. What is to be done?

Unfortunately there is no widely agreed methodology for resolving moral dilemmas or conflicts of principles. Utilitarians purport to provide such a method – choose the alternative option that will maximise welfare- but a) there is sectoral repugnance for utilitarian ethics within pluralist societies and b) even within utilitarianism itself there are major disagreements about what is to count as welfare maximisation- and in particular how to balance quantity maximisation (eg Bentham's 'greatest happiness') with distributive maximisation (eg 'of the greatest number'). In the context of health, for example, is welfare maximised if a health policy maximises the total health gain, eg the total number of QALYs gained, even if higher social classes disproportionally gain more QALYs than lower social classes, or is welfare maximised if fewer QALYs are gained but more people gain them, and especially more people in greater need gain them?

The need for *judgement* and the need for caution about the 'mathematical model of judgement'

It would be nice to be able to answer questions about how to deal with conflicting moral values or principles, and how to deal with moral dilemmas, with moral certainty or even with moral confidence, but alas I can't. What I am clear about is that in a democratic pluralist society policy makers ought to beware of answers to such questions that do purport to offer such moral certainty or even moral confidence. When agreed moral principles or values come into conflict ***judgement*** is required and unfortunately the proper approaches to carrying out such judgement are morally disputed. As the philosopher Immanuel Kant argues, 'General logic contains and can contain no rules for judgement…judgment is a peculiar talent which can be practised only, and cannot be taught'[19]. In his fascinating response to PHARMAC's questions Paul Hansen[20] suggests that PHARMAC develop an extension of a 'four step' proposal made by Hope Reynolds and Griffiths[21] whereby, after cost-utility assessment is undertaken against an agreed norm for funding, treatments that exceed this norm (in terms of cost per QALY or for year of life extension) are reviewed in relation to other moral concerns such as age, the 'rule of rescue', palliative care in terminal illness, severity of illness/health need, lack of available alternative treatment, and 'double jeopardy' as a result of co-morbidities. Unsurprisingly the Hope Reynolds and Griffiths proposal does not explain just ***how*** judgements are to be made once these various moral considerations have been addressed.

In his review Paul Hansen offers a suggestion to PHARMAC about how to carry out this last step, this moral judgement, about which I would advocate considerable 'moral caution'. Hansen notes three alternative ways for PHARMAC to deal with conflicts of value judgments: it can continue with its present policy in which value 'tradeoffs' are done by 'implicit weighting', case by case, 'in an essentially implicit and non-transparent (opaque) fashion'; it can offer some explicit criteria for 'equity weighting'; or it can develop a 'multi-criteria decision making' system such as his own in which points are agreed to be allocated in relation to an agreed range of decision criteria (such as those just mentioned). Hansen rejects the first alternative because (he implies) PHARMAC ought to be more explicit about how it comes to its funding decisions. He rejects the second because 'valid and reliable methods for estimating such weights are currently unavailable' – though he seems to approve of carrying out such equity weighting implicitly at step 4 of the four-step proposal (Hansen p21). He advocates the third alternative, the additive points system, which he points out is used internationally for a wide variety of purposes, on the grounds that such systems 'near universally. …out-perform purely intuitive decision making approaches such as PHARMAC's current approach' (Hansen, p22).

I have two concerns, one *ad hominem*, the other substantive. First, it seems to me that Hansen's rejection of the second 'equity weighting' alternative on grounds of lack of 'valid and reliable methods for estimating such weights' is equally applicable to his preferred third alternative- indeed it seems to me that his third alternative ***is*** an equity weighting system. My substantive worry however is far more important. Any ***weighting*** system begs the question of what type of system of ***judgement*** is morally appropriate for dealing with conflicting moral values. One way of judging- the one recommended by Hansen-is to assign weights to the values concerned and then add up the weights of alternative approaches to particular cases and conclude that 'the weightiest' alternative is the morally correct one. Scientists and economists are particularly attracted to this sort of approach to dealing with moral conflict, (which of course is best exemplified in utilitarian thinking), presumably because it is a mathematical approach, converting as it does moral values into numerical values and then subjecting these numerical values to mathematical analysis. One problem with such an approach is the one Hansen points out to justify his rejection of 'equity weighting' approaches, notably the lack of 'valid and reliable methods for estimating' such numerical values. The other and more major problem is the Kantian problem outlined above about proposing ***any*** rule of judgement about conflicting moral rules or values. For such judgement may in no way resemble, or be properly convertible into, a mathematical process. It may more properly be a matter of intuition, or of 'moral perception' or of consulting one's properly informed conscience. It may be more like recognising a pattern, or a harmony or beauty. It may even involve considerations, recognitions and reflections concerning virtues and vices, and the views of virtuous (and perhaps also of vicious) people. It may even be entirely 'particularist' with every judgement unique to its particular circumstances. Or it may just be inexplicable, despite being widely recognisable when it occurs. It seems unwise for PHARMAC to prejudge these deep and contentious questions about the proper method or methods for moral judgement concerning conflicting moral principles or values by deciding that the mathematical approach is the correct approach!

Jim and Pedro

A vivid and famous demonstration of the moral contentiousness of the mathematical approach to moral judgement, as exemplified by utilitarianism, is given by Bernard Williams in his story of Jim and Pedro[22]. Jim, a botanist travelling in South America, arrives in a small town market square, as Pedro is about to shoot 20 indigenous Indians so as to deter others from political protest. Pedro offers Jim, as an honoured visitor, the privilege of shooting one of the Indians and freeing the other 19. If Jim declines then Pedro will simply pursue his original plan and shoot all 20. The Indians and the local villagers beg Jim to take up the offer. Should he do so? One approach to dealing with this moral dilemma is the mathematical one that

utilitarianism would certainly use, concluding, Williams asserts, that the 'obviously right answer[s]' was that Jim should shoot one of the Indians in order to save 19. For many moral thinkers, probably including Williams, such a decision is clearly morally wrong. Yet could such rejection be morally maintained if we 'up the ante' so that the alternatives were to murder one person in order to save 100, or 1000, or a million lives?

While, like many others, I personally am attracted to the relative simplicity and clarity of the 'mathematical approach' for practical judgement in cases of moral conflict, I distrust any generalised acceptance of its use, not only because of its empirical deficit, as noted by Hansen, but also because I doubt that this sort of mathematics is **always** the morally relevant approach to moral judgment about conflicting values, even though it **sometimes** may be. Furthermore I also distrust it because I know that large numbers of conscientious moral thinkers also distrust it.

PHARMAC's question e): What if any changes do you recommend PHARMAC make to its current decision making process for HCPs ?

Thus my advice to PHARMAC is to stick with a variant of the first alternative described by Hansen, namely a variant of **implicit judgement** when moral values conflict, while making **explicit** the moral values considered to be relevant and in conflict. In pursuing this course the approach recommended by Hope Reynolds and Griffiths and commended by Hansen, but without the modifications recommended by Hansen, seems morally acceptable, makes explicit the moral considerations considered to be relevant but potentially conflicting, facilitates 'accountability for reasonableness'[23] (given some additional procedural developments proposed below), and avoids building into PHARMAC's official procedures a morally contentious mathematical, computer-based, approach to moral judgement that is likely to be vigorously, vociferously and conscientiously rejected by many.

Nonetheless I would recommend that Paul Hansen's offer of his computer programme is taken up as a **research project** with the objectives both of comparing and contrasting allocation decisions made using the Hope Reynolds Griffiths approach[21], and those that would be made if the Hansen and Ombler Point Wizard multi criteria decision analysis computer programme were used; and also to experiment with the weightings or points to be given to different criteria in order to obtain judgements that were widely acceptable in a range of hypothetical (or possibly real) scenarios.

Creation of an allocation committee

Meanwhile I would recommend three procedural adjustments. The first is that PHARMAC creates an allocation committee, drawing on such models as a clinical ethics committee, the Oxford Priorities Forum described by Hope Reynolds and Griffiths, and the NICE Citizens' Council. The role of this committee would be advisory to PHARMAC both in reviewing decisions already taken by PHARMAC and in giving advice about prospective decisions referred to it by PHARMAC where contentiousness is anticipated. In its deliberations a variant of the Hope Reynolds Griffiths four-step approach would be used and the conflicting values in particular cases would be made explicit even if the final judgements of individuals and the committee as a whole remained implicit, as they normally are 'in real life'. While I would, for practical convenience, see the benefit of accepting a norm for cost per QALY below which new products would presumptively be accepted, I would recommend against having an absolute upper value as recommended by Hansen (but not by Hope Reynolds and Griffiths). It might be that on occasion an extremely expensive but effective innovatory pharmaceutical came up for consideration whose acceptance might be recommended in the short term on the understanding that further use if successful would only be authorised if the price were to come down. It seems unnecessary to preclude this possibility.

Specifying an ethical framework

i. In addition to instituting an allocation committee it seems wise to make more explicit the ethical framework within which PHARMAC (and its allocation committee if created) makes its allocation decisions. The Beauchamp and Childress 'four principles' approach[24] is my own preferred framework[25], and has also been accepted in the UK by the National Institute for Health and Clinical Excellence[26] (and is not unlike the ethical framework adopted by Hope Reynolds and Griffiths[21]). I prefer it largely because its four prima facie moral principles (or basic values) of benefiting, not harming, respecting autonomy (so far as is compatible with equal respect for everyone's autonomy) and justice are so obviously widely acceptable, regardless of people's religious, social, cultural or philosophical background and commitments. An alternative approach worth considering is adoption of the more complex ethical framework recently approved by UNESCO in its Declaration on Bioethics and Human Rights[27]. This ethical framework has the obvious advantage of formal international approval. Unfortunately adoption of neither of these ethical frameworks will resolve the crucial problems with which I started, namely lack of agreement on a substantive theory of distributive justice. This lack of agreement in turn results from disagreement about how to deal with the conflicting moral values that, I have argued above, need to be represented in any widely acceptable substantive theory of justice; and that, as I have also argued, depends on ***judgement***. It is this area of judgement that creates much, perhaps most, of the ethical controversy about resource allocation decisions, and for which I advocate at least strongly presumptive reliance on the collective judgements of an allocation group of conscientious people drawn from a variety of perspectives and committed to trying to reach agreed decisions after having explicitly considered the moral values that they believe to be relevant but in conflict in the particular cases they are asked to consider.

Specifying the appeal mechanisms

Finally, the appeal procedures against PHARMAC's decisions ought to be specified, as recommended in Daniels and Sabins attractive 'accountability for reasonableness' framework[23]. As I understand matters there currently exist both an informal appeal process to Government via public and media opinion and a formal appeal process through the courts. The informal process may well involve vigorous appeal, often with media amplification, to the 'rule of rescue' and include the use of what is sometimes called 'shroud waving'. As indicated above my own view is that these are valid components of both the democratic process and of an acceptable substantive theory of justice. However if Government is inclined to over-rule the budget-constrained decisions of its agent PHARMAC, in response to such manifestations of public opinion, then it is surely important that Government stumps up the additional funding necessary for such 'rescues'! Otherwise Government simply adds to the opportunity costs to those whose health care needs are less obviously appealing to 'the rule of rescue'. The formal legal appeal process should continue to be empowered to reverse decisions by PHARMAC, but only if there has been a demonstrable failure to pursue the agreed processes, or if some relevant new evidence is adduced. The courts should continue to eschew any power to reverse an allocation decision made by PHARMAC simply because they disagree with it!

Final comment: Be prepared for moral dissatisfaction

Finally let me reiterate the need for those involved in allocation of scarce resources to expect moral dissatisfaction with their recommendations, no matter how conscientiously and assiduously derived. The fact that resources are limited entails that not all the competing claims will be met. Those claims will often, perhaps usually, have some moral justification. Thus there is likely to be moral dissatisfaction with ***any*** outcome, for any outcome will generally involve the over-riding of claims that have some moral justification, in favour of

other claims judged stronger in the particular circumstances. There is, I'm pretty sure, no generally acceptable way of avoiding this dissatisfaction; indeed perhaps it would be morally worrying if there were, for absence of such dissatisfaction might well signal a loss of moral sensitivity to failure to meet morally justified claims- no matter how justified that failure is in the particular circumstances.

Notes and references

[1] Gillon R. Philosophical medical ethics. Chichester: Wiley, 1985 and 13 subsequent reprints, latest 2003.

[2] See entry on Distributive Justice by Julian Lamont at http://plato.stanford.edu/entries/justice-distributive (last accessed 27 july 2005).

[3] see entry on justice as a virtue by Michael Slote at http://plato.standford.edu/entries/justice-virtue (last accessed 20 august 2005).

[4] Gillon R Value judgments about equity in health. In: Oliver A, Cookson R, McDaid D (eds): The issues panel for equity in health- discussion papers. London: The Nuffield Trust, 2001.

[5] For example in Sen AK. Inequality re-examined. Cambridge, Mass: Harvard University Press, 1992. As noted, Sen's preferred answer is (roughly) equality of capability to achieve what one values.

[6] Though I should add that some economists have made heroic efforts to oppose this norm, either by contorting the meaning of 'need' to turn it into 'capacity to benefit'- but of course people can be in enormous need and yet not have a capacity to benefit, as for example when they need a cure for their fatal disease but such a cure doesn't exist; or by arguing that 'needs assessment is based on faulty logic- the faulty logic of the imperative of "the size of the problem". That faulty logic needs to be exposed – and exposed again. It is so pervasive in health care. The fact that it is pervasive however is no reason for believing that it is in any sense right'- Devlin N, Hansen P. Allocating Vote Health- 'Needs Assessment' and an Economics-Based Approach. Treasury Working Paper 00/4, section 2. At: www.treasury.govt.nz/workingpapers/2000/00-4. Last accessed 11.12.05. I fail to discern the 'faulty logic'- it seems rather a disagreement about the premises to be used in arguments rather than their logic. However since PHARMAC's remit is clearly acknowledged by Hansen, in his report corresponding to this one, to require a component of needs-based analysis, and since I shall be arguing that needs based analysis is not the only relevant criterion for fair allocation of PHARMAC's resources, I shall not pursue my argument against whole hearted rejection of the criterion of need any further here. See also my Note 20 below.

[7] Doyal L, Gough I. A Theory of human need. Basingstoke: MacMillan Press, 1991.

[8] This synoptic account is informed by the work of David Wiggins in his Needs, values, truth.. Oxford: Blackwell, 1987 and differs from Roger Crisp's broader account of need in which any advancement of human wellbeing can create or is a response to a need. See Crisp R. Treatment according to need: justice and the British National Health Service In: Rhodes R, Battin M, Silvers A (eds). Medicine and social justice. Oxford: Oxford University Press, 2002, pp 134-143. There is of course an elliptical sense of need in which anything can be a need if without it some prior purpose is frustrated- if I am to go to the dinner tonight I will need a dinner jacket. By my Wiggins-informed account, only if I will be harmed by not going to the dinner do I have a need for the dinner jacket- and the greater the harm the greater that need.

[9] One of the earliest and most trenchant critiques is given by Harris J. QALYfying the value of life. Journal of Medical Ethics, 1987; 13: 117-123.

[10] A review of the debate about QALYs is given by Schwappach D. Resource allocation, social values and the QALY: a review of the debate and empirical evidence. Health Expectations, 2002; 5: 210-222.

[11] I have not in this paper considered 'desert' in the sense of merit or relative virtue, or absence of vice, as a criterion for fair allocation of scarce health care resources as my own analysis tends to eliminate this criterion as unacceptable for health care justice. Thus for example the idea that people who bring about their own ill health should not be given medication that would meet their needs because they 'don't deserve it' is hard to sustain at all, but in any case would require radical legislation before it could become relevant to PHARMAC. This does not of course rule out use of the 'sufficient benefit' criterion to prioritise, for example, provision of liver transplants to non- drinkers, including those who have given up alcohol as a demonstration of their ability and intention to stop drinking, ahead of drinkers who are unlikely to stop drinking and are thus likely to gain far less benefit from such a transplant. Similar considerations would apply to pharmaceutical treatments for which life style changes would be required for the treatment to be able to provide sufficient benefit.

[12] Jonsen A. Bentham in a box: technology assessment and healthcare allocation. Law medicine and health care 1986; 14: 172-174.

[13] Hadorn D. Setting Health Care Priorities in Oregon- Cost-effectiveness Meets the Rule of Rescue. JAMA 1991 vol 265: pp 2218-2225.

[14] see for example Jakobovits I. Jewish medical ethics. New York: Bloch Publishing Co, 1959, p45 where Dr Jakobovitz refers to both Talmudic and Koranic claims that to save a single life is equivalent to saving a whole world, or humanity as a whole.

[15] End of life decisions. www.ewtn.com/expert/answers/end_of_life_decisions.htm. Last accessed 3.12.05.

[16] Richardson J, McKie J. The rule of rescue. www.buseco.monash.edu.au/centres/che/pubs/wp112. Last accessed 4.12.05.

[17] Jonsen AR. . Bentham in a box: technology assessment and health care allocation. Law medicine and health care, 1986; 14: 172-174.

[18] Jonsen A. Bentham in a box; technology assessment and health care allocation. National Forum [USA] 22/9/89.

[19] Kant I. Critique of pure reason A132-A133. See for example Norman Kemp Smith's translation published in London: Macmillan Press, 1973 print, pp 177-178. Kant's argument, in summary, is that since judgement is 'the faculty of subsuming under rules' there can be no general rule for judgement on pain of an infinite regress; for every time the requirement for judgement between rules arises there will be the need to judge which rule should apply, one or other of the conflicting rules or the purported rule for judgement between them – and if there were a rule for that judgement, the same problem would arise, with the need for a further rule of judgement and so on ad infinitum.

[20] His response deserves a full response, which I am alas unable to provide at this time. Suffice it to note that I believe his rejection of a needs based approach to resource allocation, largely implicit in his response but vigorous in his and Nancy Devlin's Treasury Working Paper 00/4, is mistaken, as I indicate in Note 6 above. To his credit he does however offer a way of accommodating such a needs based approach within a fixed budget (his value judgment 4 at p 13 of his response, corresponding to point p on his figure 2at p11). It is an approach that I personally would be keen to see pursued, while acknowledging that it is heavily 'weighted' to the moral value of distribution in proportion to healthcare needs; and while acknowledging the general opacity of 'judgement' between conflicting moral values.

[21] Hope T, Reynolds J, Griffiths S. Rationing Decisions: Integrating Cost-Effectiveness with Other Values. In: Rhodes R, Battin M, Silvers A (eds). Medicine and Social Justice. Oxford, New York: Oxford University Press, 2002, pp 144-155.

[22] Williams B. A critique of utilitarianism. In: Smart J, Williams B. Utiltarianism for and against. Cambridge: Cambridge University Press, 1973, pp 75-155, the Jim and Pedro example at pp 98-100.

[23] Daniels N, Sabin J. Setting Limits Fairly. Oxford, New York: Oxford University Press, 2002, pp43-66.

[24] Beauchamp T, Childress J. Principles of Biomedical Ethics. Oxford, New York: Oxford University Press 2001 (5th ed).

[25] My own use of this approach is described in reference 1 above and summarised in: Gillon R. Medical Ethics: Four principles plus attention to scope. British Medical Journal, 1994; 309: 184-188.

[26] National Institute for Health and Clinical Excellence. Social value judgements – principles for the development of NICE guidance. At: www.nice.org.uk/pdf/social_valuejudgement-08_12_05.pdf. Last accessed on 29.12.05.

[27] Universal Declaration on Bioethics and Human Rights. At: www.unesco.org/en/ev.php-URL_ID=30274& URL_DO=DO_PRINTPAGE&URL_SECTION=201.html - (or more easily accessed via a Google search!)- last accessed on 11.12.05.

A theoretical review of PHARMAC's over-arching approach to deciding which pharmaceuticals to fund, including high cost ones

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May 2006

Executive Summary

- All approaches to deciding which pharmaceuticals to fund, including high cost ones, are inherently *normative* in nature. Such decisions depend on decision makers' (and their constituencies') value judgements (or ethical positions), which, in general terms, depend on their theories or beliefs about social justice/equity.

- As it should be in my opinion, PHARMAC's over-arching approach to such decision making is *economics*-based, and heavily informed by cost-utility analysis (CUA). In essence, the objective of economics-based decision-making approaches is the maximisation of 'value for money'. But the key question is, What does 'maximising value for money' mean in the present context?

- Logically, one thing that it does *not* necessarily mean is maximising the total number of Quality-Adjusted Life Years (QALYs) gained from PHARMAC's pharmaceuticals budget. This would correspond to a particular interpretation of 'value' — one that reflects the particular value judgement (ethical position) of utilitarianism. In fact, other value judgements — of which, in theory, there is an infinite number available (e.g. Rawlsianism, 'rule of rescue', 'fair innings', etc.) — may be more acceptable (or not) to decision makers and their constituencies.

- Similarly, the application of CUA is *not* synonymous with maximising the total number of QALYs gained. CUA's proper role is to provide information about the costs per QALY (gained) for different pharmaceuticals and patient groups. How PHARMAC uses this information is a separate matter that depends on the value judgements with respect to the interpretation of 'value for money' that are adopted.

- In my opinion, the challenge (not to be under-estimated) facing PHARMAC's decision makers is to better represent their preferred value judgements (and those of PHARMAC's constituencies) by clearly articulating valid criteria, and their relative importance, for deciding which pharmaceuticals to fund (including high cost ones).

Accordingly, three recommendations are offered here to PHARMAC:

1. That PHARMAC be more explicit and transparent about its over-arching approach to deciding which pharmaceuticals to fund. A 'four-step approach' that could form the basis for such a declaration is explained in this review (see pp. 15-17).

2. That PHARMAC considers 'tightening up' how it expresses its decision criteria, at least as they are written in its *Operating Policies and Procedures*. (PHARMAC may want also to consult more fully its constituencies on what these criteria should be.)

3. That PHARMAC considers whether it wants to continue determining the relative importance of its decision criteria in an 'implicit' (intuitive) fashion or, alternatively, uses more formal methods available from the field of Multiple Criteria Decision Analysis (explained here; see pp. 21-5). In my opinion, further research (and experimentation) into the usefulness of these methods for PHARMAC would be worthwhile.

1. Introduction to the review and acknowledgements

PHARMAC commissioned this review, "containing an evaluation of the funding of High Cost Pharmaceuticals" (defined below) and answers to these eight questions:

(a) What are the main economic/social justice/ethical theories relevant to how decisions on funding 'high cost' pharmaceuticals could be made?

(b) What, if any, justification is there for assessing High Cost Pharmaceuticals differently from other pharmaceuticals considered for public subsidisation?

(c) What might be the downsides of valuing High Cost Pharmaceuticals differently?

(d) Could cost-utility analysis be used more effectively when considering 'high cost' pharmaceuticals? If so, how?

(e) What, if any, changes do you recommend PHARMAC make to its current decision-making process for 'high cost' pharmaceuticals (and others too)?

(f) What role should 'rule of rescue' play in assessing High Cost Pharmaceuticals for funding?

(g) What are the arguments for and against paying a higher price (per QALY gained, for example) for pharmaceuticals for those who are worse off clinically with poor quality-adjusted life expectancy, but of arguably greater need (for example, the terminally ill)?

(h) Are there any general comments that you wish to make?

So-called "High Cost Pharmaceuticals" are ones for which their "cost per quality-adjusted life year (QALY) … is significantly higher than the less than $10,000/QALY of most Pharmaceutical Schedule listings." (PHARMAC 2003, pp. 1-2). Two examples of high cost pharmaceuticals are Pulmozyme and Beta-interferon,[1] which cost between $41,000 and $81,000 per QALY.

Although PHARMAC's questions relate to the funding of *high cost* pharmaceuticals in particular, the relevant theoretical issues apply to the funding of *all* pharmaceuticals in general (i.e. both high and lower cost). Hence this is a theoretical review of PHARMAC's over-arching approach to deciding which pharmaceuticals to fund, including (but not limited to) high cost pharmaceuticals.

Consistent with the main tenor of PHARMAC's questions, this review is concerned primarily with *distributional* rather than *procedural* justice. That is, the focus is on the relative desirability of alternative possible allocations of PHARMAC's pharmaceutical budget rather than the relative desirability of alternative processes by which such allocations are reached.[2]

PHARMAC's eight questions are split between the following two sections. Section 2 (questions a - c, h) considers the theoretical foundations to approaches to deciding which pharmaceuticals to fund (including high cost ones). Based on these foundations, Section 3

[1] Other examples are Cerezyme, Glivec and growth hormone.

[2] Of course, this is not to deny that procedural justice is also important. Some of the commentators on this review (acknowledged below) raised issues associated with procedural justice, especially the importance of greater involvement by Māori, as well as immigrant communities, in the pharmaceutical funding decision-making process. In addition, Andrew Moore directed PHARMAC's attention to Daniels & Sabin's (1998) (and see Daniels 2000) "accountability for reasonableness" doctrine: the essentially common-sense elements of a decision-making process that Daniels and Sabin regard as being necessary to ensure the process' legitimacy and credibility.

(questions d - g, h) discusses possible improvements to PHARMAC's over-arching decision-making approach. The review closes with its conclusion and recommendations.

It is probably worthwhile acknowledging here at outset that, consistent with my training as an Economist, the main themes developed in this review are derived from economic theory (particularly Welfare Economics), and also my knowledge of practical methods for priority setting and decision making. I am not a trained moral philosopher or ethicist. Nonetheless, Welfare Economics does, by definition, encompass ethics, especially with respect to providing a framework of analysis.

Acknowledgements

I am grateful to the staff at PHARMAC for their insightful comments and suggestions as this review has progressed through its several iterations.

In addition, this final version has benefited from my reading of the commentaries commissioned by PHARMAC from the following nine individuals on my review and another by Raanan Gillon (Emeritus Professor of Medical Ethics, Imperial College London), who also commented on my review. Naturally, any remaining errors are my responsibility alone.

Toni Ashton (School of Population Health, University of Auckland)

Sandra Coney (PHARMAC Consumer Advisory Committee)

Matiu Dickson (School of Law, University of Waikato)

David Hadorn (Law & Economics Consulting Group)

George Laking (Wolfson Molecular Imaging Centre, University of Manchester)

Robert Logan (Hutt Valley District Health Board)

Nicholas Mays (London School of Hygiene & Tropical Medicine, University of London)

Andrew Moore (Department of Philosophy, University of Otago)

Martin Wilkinson (School of Population Health, University of Auckland)

2. Theoretical foundations to deciding which pharmaceuticals to fund, including high cost ones

(PHARMAC's questions pertaining to this section)

(a) What are the main economic/social justice/ethical theories relevant to how decisions on funding 'high cost' pharmaceuticals could be made?

(b) What, if any, justification is there for assessing High Cost Pharmaceuticals differently from other pharmaceuticals considered for public subsidisation?

(c) What might be the downsides of valuing High Cost Pharmaceuticals differently?

(h) Are there any general comments that you wish to make?

Which value judgements from the infinite number available?

All approaches to deciding which pharmaceuticals to fund, including high cost ones — and, correspondingly, which pharmaceuticals not to fund — are inherently *normative* in nature. Such decisions depend on decision makers' (and their constituencies') value judgements (or ethical positions), which, in general terms, depend on their theories or beliefs about social justice/equity.

For example, decision making (e.g. 'a rule') that automatically disqualifies relatively high cost pharmaceuticals (i.e. with a high cost per Quality-Adjusted Life Year (QALY)[3] gained), in favour of lower cost pharmaceuticals (with a lower cost per QALY gained), by definition, seeks to maximise the total number of QALYs gained from a given amount of spending (PHARMAC's pharmaceuticals budget). Such decision making corresponds to a particular value judgement (ethical position): *utilitarianism* — whereby each QALY gained is regarded as being of equal value regardless of to whom it accrues, and all that matters is the total number of QALYs gained (in other words, one person's QALY gains are regarded as being as valuable as any other person's).[4]

As is well-known, however, the utilitarian value judgement is not regarded as being 'fair' and 'reasonable' by everyone. Other value judgements may be more acceptable (or not) to decision makers and their constituencies.

For example, probably the most well-known, and popular (Brock 2002), alternative to the utilitarian value judgement is *Rawlsianism*. In the present context, the Rawlsian value judgement favours patient groups with relatively poor health (e.g. in QALY terms) over groups with better health, such that, all else being equal, pharmaceuticals benefiting the former should be funded in preference to pharmaceuticals benefiting the latter, with the ultimate objective being the equalisation of the respective groups' health. As is demonstrated theoretically later below, Rawlsianism is likely to have a very different outcome in terms of the total number of QALYs gained from PHARMAC's budget than the utilitarian value judgement (i.e. the number of QALYs gained will *not* be maximised).

[3] QALYs are described very briefly under the heading "Cost-utility analysis" below. Detailed information about them is available from Drummond et al. (1997), for example.

[4] Strictly speaking, though, the focus of utilitarianism is the maximisation of 'utility' or welfare (i.e. happiness/satisfaction). Clearly, QALYs are not the same as happiness (to an individual or society) — because other 'things' (other than health) contribute to happiness as well. As discussed below under the heading "Cost-utility analysis" (CUA), such an exclusive focus (as for CUA) on health status, typically measured in terms of QALYs, is often referred to as being '*extra*-welfarist' because it supplants the conventional social welfare focus.

Although both of these value judgements are very well known, utilitarianism and Rawlsianism are but two of an infinite number of value judgements that are available in theory (other popular ones are discussed later in the review). Many of them would support the funding of high cost pharmaceuticals in preference to lower cost ones, and many would not.

The challenge for PHARMAC's decision makers is to choose the 'best' value judgement(s) to apply (itself a value judgement!) and to operationalise them in PHARMAC's decision making. The objective of this section of the review is to lay a theoretical foundation for thinking about this problem.

A variety of possible approaches to decision making

In general, decision making with respect to which pharmaceuticals (and other types of publicly-funded health care)[5] to fund can be implemented via 'technical' or 'non-technical' approaches.

In brief, *technical* decision-making approaches centre on the use of information and processes that result in funding decisions being made in an explicit, transparent, consistent, impartial and dispassionate fashion.[6] In contrast, *non-technical* decision-making approaches are less explicit and transparent in nature. Although not intrinsically so, such approaches often tend to rely on political expediency and lobbying by interest groups and ad hoc decision making favouring the status quo. An example of such decision making is PHARMAC's 'decision' to fund Beta-interferon, "under Ministerial direction" (PHARMAC 2003, p. 2).

In addition, technical decision-making approaches are either based on economics principles or not. A widespread example of a *non-economics*-based decision-making approach is 'needs assessment' whereby priorities are determined according to identified health 'needs' (with all the difficulty attending the meaning of this term). These priorities are determined without regard to the relative costs of their being met, and sometimes without regard to which needs are able to be modified by treatment (depending on how 'need' is measured). For a critique of needs assessment in the context of New Zealand's publicly-funded health and disability services in general, see Devlin & Hansen (2000).

In contrast, the defining characteristic of *economics*-based (technical) decision-making approaches is that the outcomes of alternative funding allocations are compared relative to each other, with the explicit objective of maximising the *value* of the *benefits* realised (both variously defined) from the money that is spent. The objective, in essence, is to maximise the 'value for money' from a given amount of spending.

As it should be in my opinion, most of PHARMAC's decision making is economics-based (e.g. with the exception of the above-mentioned decision to fund Beta-interferon). In particular, PHARMAC's decision-making approach is heavily informed by cost-utility analysis (PHARMAC 2004).

Cost-utility analysis

As is well-known, cost-*utility* analysis (CUA) is a refined form of cost-*effective* analysis (CEA), which is itself a modified form of cost-*benefit* analysis (CBA). The modification is that instead of reductions in mortality and morbidity ('the benefits') being valued in monetary terms, they are left in their natural units of measurement (life-years, etc.), which are then adjusted for their *health-related* quality of life to get QALYs for CUA.

Although CUA is a descendant of CBA, it has a different objective. Whereas CBA focuses on social welfare, consisting exclusively of individual 'utilities' or 'welfare' (i.e.

[5] Parts of this section (Section 2) are adapted from Devlin & Hansen (1999, 2000).
[6] Thanks to commentator George Laking for reminding me of these last two "virtues".

happiness/satisfaction (however this is aggregated across individuals), CUA concentrates on health status, typically measured in terms of QALYs. Because it supplants CBA's conventional social welfare focus, CUA is often referred to as being '*extra*-welfarist'.

Also, the preferences that people might have about *how* health care funding decisions are made, and also about how health services are *delivered*, tend to be ignored by CUA (other than any links that might exist between these preferences and service effectiveness and health outcomes). In other words, and as noted in the Introduction, consistent with the focus of this review, CUA is concerned primarily with distributive justice rather than procedural justice.

"The best health outcomes … from within the amount of funding provided"

PHARMAC's stated 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*." (PHARMAC 2001, p. 2; my italics).

What does "the best health outcomes" mean in this context? An interpretation that is consistent with the preceding discussion is that they are the health outcomes, as defined in terms of QALYs gained (by "eligible people in need of pharmaceuticals"), that maximise the 'value for money' from PHARMAC's pharmaceuticals budget. But what does "maximising the 'value for money' from PHARMAC's pharmaceuticals budget" mean?

Logically, one thing that it does *not* necessarily mean is maximising the total number of QALYs gained. This would correspond to a particular interpretation of 'value' — as discussed earlier, one that reflects the utilitarian value judgement (whereby the total number of QALYs gained is all that matters, without regard to whom they accrue). As noted earlier, other value judgements may be more acceptable (or not) to decision makers and their constituencies.

Not necessarily the same as maximising QALYs gained

Similarly, CUA is *not* synonymous with maximising the total number of QALYs (gained) from a given amount of pharmaceutical spending. All that CUA does is provide information about the costs per QALY for different pharmaceuticals and patient groups. How that information is used for decision-making purposes is a separate matter.

For example, the (US) Panel on Cost-Effectiveness in Health and Medicine recommends, in general terms, that CUAs "are an aid to decision making, not a complete procedure for making decisions, because they cannot incorporate all the values relevant to the decisions." (Gold et al. 1996, p. 22). Likewise, the UK's National Institute for Health and Clinical Excellence (NICE) recommends that CUA "in the economic evaluation of particular interventions is a necessary, but insufficient, basis for decisions about cost-effectiveness." (NICE 2005, Recommendation 4, p. 3)

In particular, CUA does *not* reveal how the QALYs gained from a given amount of pharmaceutical spending ought to be compared across different pharmaceuticals and patient groups such that, relative to alternative funding allocations, the QALY tradeoffs between pharmaceuticals and patient groups are optimal. Such QALY comparisons and aggregations inevitably require value judgements by decision makers (reflecting their beliefs about social justice/equity) — such as, but not necessarily, the utilitarian value judgement or, but again not necessarily, the Rawlsian value judgement, etc.

It is clear that PHARMAC accepts these principles implicitly. In some of its writings, however, it employs a narrower definition of CUA than the description above. According to PHARMAC (2004, p. 10; my italics): "The purpose of CUA is to guide decisions aimed at *maximising the number of QALYs* for a given amount of money." In other words, according to PHARMAC, CUA *is* based on utilitarianism. (This is incorrect.)

Nonetheless, PHARMAC explicitly acknowledges that after having ranked pharmaceuticals according to their costs per QALY, other value judgements (e.g. Rawlsianism, etc.) might be bought to bear. PHARMAC (2004, p. 7) summarises the role of CUA in its decision-making approach thus:

> CUA is a tool used for maximising health. CUA cannot explicitly assist in any debate about the ethics of maximising health compared to treating the "needy" [for example]. CUA does clarify the size of the efficiency trade-off if a decision to treat the needy is made (where the needy will gain less benefit per dollar spent than patients who would benefit from an alternative proposal). However, this is the only exception where CUA informs ethical debate.
>
> For this reason, we have adopted CUA as one part of our decision-making framework. Decisions can, and have, been made to treat the needy on grounds other than maximising health. In short, CUA results are considered a guide to decision making, not a substitute.

Although this approach is essentially correct in terms of the final decisions likely to be reached, I think PHARMAC's statements like the one reproduced above ("The purpose of CUA is to guide decisions aimed at maximising the number of QALYs for a given amount of money") are unhelpful.

It is better instead to keep the estimation of costs per QALY gained separate from the application of value judgements for comparing and aggregating them and ultimately deciding which pharmaceuticals to fund. In other words, CUA should be value judgement free. Utilitarianism, or any other value judgement for that matter, is not intrinsic to CUA (even if, in practice, the utilitarianism implicit in PHARMAC's implementation of CUA is later over-ridden by other value judgements to reach a final decision).

A theoretical illustration of the inevitability of value judgements

The remainder of this section consists of an illustration via a stylised example of the inevitability of value judgements when deciding which pharmaceuticals to fund. The logical implications of the value judgement of economic efficiency and four other common examples are demonstrated.

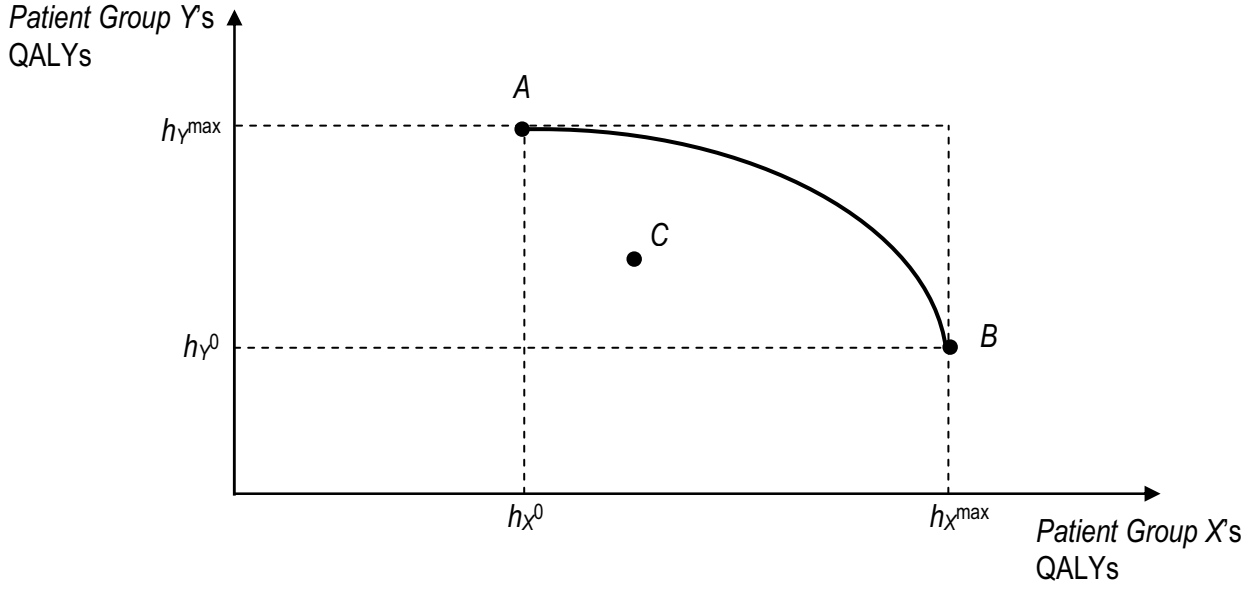
Although some of the concepts presented here may seem arcane to some readers, understanding them is worthwhile as they are fundamental to the theoretical basis of allocative decision making in general. These concepts, including the model used to illustrate them here,[7] are fundamental to Welfare Economics (the framework for studying the social desirability of alternative allocations of goods and services in general).

The diagram in Figure 1 below represents alternative combinations of health, as measured in QALYs (i.e. incorporating both life expectancy and health-related quality of life), that are possible for two patient groups, referred to simply as *Patient Group X* and *Patient Group Y*. Each patient group can be treated with one drug only: *Patient Group X* with *Drug X* and *Patient Group Y* with *Drug Y*.

If *Patient Group X* were not treated with *Drug X*, then the health of this patient group would correspond to just h_x^0 QALYs in the diagram. Similarly, without *Drug Y*, *Patient Group Y* would enjoy just h_y^0 QALYs. Thus *Patient Group Y* can be said to have a greater 'need' for treatment than *Patient Group X* in the sense that without treatment the former has less health than the latter (i.e. $h_Y^0 < h_X^0$).

[7] Details of the model's derivation appear in Appendix A, which is closely adapted from Devlin & Hansen (2000), and the diagrams are virtually identical to ones in Culyer & Wagstaff (1993) and Culyer (1995).

Figure 1: The 'health possibilities (efficiency) frontier' for Patient Groups X and Y



For simplicity (it does not affect the essence of the following arguments), let's assume that *Patient Group X* and *Patient Group Y* are of the same size (e.g. 10,000 people each) and that *Drug X* and *Drug Y* cost the same per unit (e.g. $1000 each per patient per year). (As explained below, the patient groups have different 'capacities to benefit' from treatment with the respective pharmaceuticals.) The problem facing PHARMAC is to decide how to split its available pharmaceuticals budget (e.g. $10 million) between the two pharmaceuticals (patient groups).

Again with reference to Figure 1, if PHARMAC were to spend all of its budget on *Drug Y* alone, then *Patient Group Y* would end up with h_Y^{max} QALYs and *Patient Group X* would be stuck with h_X^0. This is point *A* in the diagram. Alternatively, if PHARMAC were to spend all of its budget on *Drug X* alone, then *Patient Group X* would end up with h_X^{max} QALYs and *Patient Group Y* with h_Y^0. This is point *B* in the diagram.

Thus, in contrast to their relative health needs (where, as noted above, *Y* is 'needier' than *X*; i.e. $h_Y^0 < h_X^0$), *Patient Group X* has a greater capacity to benefit from treatment than *Patient Group Y* (i.e. $h_X^{max} - h_X^0 > h_Y^{max} - h_Y^0$). (This contrast between the patient groups' 'needs' and 'capacities to benefit' is deliberate here, as it serves, as we will see, to most starkly illuminate the differences between common value judgements adopted by decision makers.)

Also, although *Drug X* and *Drug Y* are assumed to cost the same (per unit of pharmaceutical), because *Drug Y* has the potential to produce fewer QALYs than *Drug X* (i.e. as above, $h_X^{max} - h_X^0 > h_Y^{max} - h_Y^0$), *Drug Y* costs more *per QALY gained* than *Drug X* does. Thus in this example, *Drug Y* can be said to be the 'high cost' pharmaceutical.

As explained above, points A and B in Figure 1 correspond to extreme allocations of PHARMAC's budget: all or nothing spent on *Drug Y* or *Drug X* respectively. Furthermore, the curve between points *A* and *B* represents all maximum combinations of health (in QALY terms) for the two patient groups, corresponding to all possible allocations of PHARMAC's budget between *Drug X* and *Drug Y*, that are affordable with PHARMAC's budget. This curve is often referred to as the 'health possibilities frontier', or, as we will see later below, the 'health efficiency frontier'.

The negative slope of the health possibilities frontier in Figure 1 arises from the fact that given PHARMAC's budget is fixed (and PHARMAC must 'live within' it) then in order for one

patient group to consume more of its particular pharmaceutical (thereby gaining QALYs) the other group must reduce its consumption of its pharmaceutical (thereby losing QALYs). In other words, given PHARMAC's budget constraint, there is a trade-off between *X*'s QALY gains and *Y*'s QALY gains (i.e. as we 'move' along the frontier) — to have more of one, there must be less of the other, and vice versa (i.e. the 'opportunity cost' of one is the other).[8]

PHARMAC's 'problem' is to decide which point on the health possibilities (efficiency) frontier is 'the best' — that corresponds to PHARMAC's objective of "securing the best health outcomes ... from within the amount of funding provided" (as above) or, in other words, that represents the maximum value for money. (Having decided on the best allocation of QALY gains to *Patient Group X* and *Patient Group Y* respectively, it is conceptually simple to determine the corresponding division of PHARMAC's budget between *Drug X* and *Drug Y*.[9]) As we will now see, however, PHARMAC's decision will require a stronger value judgement than (mere) economic efficiency.

Economic efficiency is never enough on its own

For all resource allocation problems in general, including PHARMAC's funding decisions in particular, the *only* value judgement that is virtually universally accepted as being 'fair' and 'reasonable' is that the allocation should be economically efficient (also referred to as being 'Pareto' efficient[10] by economists). Equivalently, most people would agree that the allocation should not be inefficient, as inefficiency implies wastage.

With reference to the example above, an inefficient allocation of PHARMAC's budget between *Drug X* and *Drug Y* is one for which it would be possible to reallocate the budget (without having to increase it; in other words, staying within PHARMAC's budget) such that at least one of the patient groups would gain QALYs without the other group losing QALYs.

For example, with reference to Figure 1 again, suppose such a reallocation resulted in the patient groups moving from point *C* (corresponding to an initial hypothetical allocation) in a *north-easterly* direction to anywhere on the corresponding portion of the health possibilities frontier (as explained earlier, representing all maximum combinations of QALYs for the patient groups that are affordable with PHARMAC's budget). This would represent an *increase in efficiency* (or a 'Pareto improvement'). Most, but not necessarily all,[11] people would agree with the value judgement that an increase in efficiency (an 'efficiency gain') is a 'good thing', as at least one person (or patient group) is being benefited without anyone else being harmed.

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[8] More specifically, though, why is the 'health possibilities frontier' curved (like a banana) towards the diagram's origin rather than a straight line? The frontier's curvature represents the fact that the trade-off between *X*'s QALY gains and *Y*'s QALY gains (i.e. their opportunity costs) is increasing. For example, to get more QALY gains for *Patient Group X* requires ever-increasing sacrifices of QALY gains for *Patient Group Y*. Fundamentally, this (increasing) rate of exchange between *Patient Group X's* QALYs and *Patient Group Y's* QALYs is determined by the relative effectiveness of the two pharmaceuticals at producing QALYs for each patient group and the size of PHARMAC's budget. The nature of these relationships (merely stated here) is clarified via the detailed derivation of the frontier presented in Appendix A, which despite containing 'additional material', includes several (economic) concepts that are relevant (but not essential) to thinking about allocative decision making in the present context.

[9] For details, for example, see Devlin & Hansen (2000). Alternatively, you should be able to figure out how to split PHARMAC's budget between *Drug X* and *Drug Y* yourself from the model presented in this review's Appendix A.

[10] Named after Vilfredo Pareto (1848-1923), an Italian economist and sociologist, who introduced the concept.

[11] In particular, a person with a (strict) egalitarian ethical position might disagree. To an egalitarian, an allocation of pharmaceutical spending that makes someone who is already relatively healthy even more healthy will not be desirable, even if someone in relatively poor health is not harmed, because this increases inequality in the distribution of health.

An allocation is said to be *efficient* if no further efficiency gains (or Pareto improvements) are possible — i.e. when no further QALY gains to either or both patient groups are possible without at least one of the groups losing QALYs. Thus it should be obvious that *all* points on the health possibilities frontier are economically efficient (compared to all points to the left of or below the frontier, which are inefficient). In other words, for any point on the frontier it is impossible to achieve further QALY gains for either or both patient groups without at least one of the groups losing QALYs (i.e. by moving along the frontier — given that anywhere to the right of or above the frontier is unaffordable given PHARMAC's budget). For this reason, as mentioned earlier, the 'health possibilities frontier' is also referred to as the 'health efficiency frontier'.

The value judgment that a budget allocation should be economically efficient (anywhere on the health possibilities frontier) is broader than the value judgment of utilitarianism. As explained earlier, the utilitarian allocation is the one that maximises the total number of QALYs gained, which as we will demonstrate below, corresponds to a particular point on the frontier.[12]

Hence, from a policy-making perspective, the problem is that the value judgement that economic efficiency is desirable (and that inefficiency is not) — which most people would agree with — does not distinguish between (the infinite number of) points on the 'health possibilities (efficiency) frontier'. All points on the frontier are economically efficient!

A stronger value judgement than economic efficiency is required

Therefore PHARMAC cannot appeal to the criterion (value judgement) of economic efficiency alone to help it decide how 'best' to allocate its pharmaceuticals budget. An additional value judgement, and one that is stronger than economic efficiency — and therefore one that is inevitably less universally accepted — is required. Specifically, a value judgement that identifies QALY tradeoffs between patient groups that are deemed acceptable, or 'equitable' or 'distributionally just' as such aggregations are more commonly known, is required.

As noted earlier, an infinite number of such value judgements is possible in theory — one for each and every point on the health possibilities (efficiency) frontier! However, particular 'types' of value judgement are generally more popular (e.g. morally acceptable or relevant) than others. (Examples of value judgements that would be regarded by most people as being morally unacceptable or irrelevant include ones based on a person's racial characteristics, political beliefs, height, or eye colour, etc.)

The following four value judgements are mentioned relatively often in the 'heath economics' literature (e.g. see Williams & Cookson 2000 and Cuadras-Morato et al. 2001), and are focussed on here because they are amenable to diagrammatic representation via the apparatus introduced in Figure 1. Other relatively common value judgements (less amenable to diagrammatic representation) are discussed in the following section. Please note, the purpose of the following discussion is not to advocate any particular value judgement over others, but rather to compare and contrast the logical implications of these four common examples.

[12] NICE (2005) mistakes economic efficiency for utilitarianism. Presumably this reflects a confusion between economic (Pareto) efficiency (as discussed above) and *potential* Pareto efficiency — whereby those who gain QALYs *could* compensate (but need not actually do so) those who lose QALYs and still be better off (hence total QALYs gained are to be maximised).

Value judgement 1: Utilitarianism (or 'Benthamism')

As discussed earlier, this value judgement asserts that each QALY gained is of equal value regardless of to whom it accrues, such that all QALYs are perfect substitutes for each other, and all that matters is the total number of QALYs gained. More generally, this value judgement corresponds to Jeremy Bentham's,[13] "the greatest happiness principle".

With respect to the diagrammatic apparatus introduced in Figure 1, the utilitarian value judgement dictates that the total number of QALYs produced from PHARMAC's budget be maximised, regardless of to which patient groups they accrue. In other words, *Patient Group X's* QALYs and *Patient Group Y's* QALYs are treated as being perfect substitutes, and it is simply a matter of choosing the unique pair from the health possibilities (efficiency) frontier that maximises their sum, *Patient Group X's* QALYs + *Patient Group Y's QALYs.*

Diagrammatically (see Figure 2 below), this unique pair is identified by the point of tangency — point *U* — of the health possibilities (efficiency) frontier to a line[14] with slope = –1 (i.e. at 45˚ from both axes). The equation of this line is *Patient Group X's* QALYs + *Patient Group Y's QALYs* = 'some particular number of QALYs' (e.g. 1000). Clearly, as you 'move' along this line, a reduction in the number of QALYs for one patient group is exactly offset by an increase in the number for the other group (hence the line's slope = –1).

Note that *if* other parallel lines were to be drawn in Figure 2, then the further from the origin a given line was, the greater the total number of QALYs; however, unless the line *touches* (is tangent to) the health possibilities frontier, then that total number of QALYs is unaffordable with PHARMAC's budget. Thus the greatest total number of QALYs that is affordable is found at the tangency of the actual line in Figure 2 with the frontier (point *U*).

Thus the utilitarian allocation is identified as point *U* in Figure 2. As for the three other value judgements below, the QALYs for the two patient groups can be identified by tracing them off the diagram's axes (not shown in the figure).

Value judgement 2: Rawlsianism (or 'maximin')

As explained earlier, the Rawlsian value judgement favours patient groups with relatively poor health over groups with better health, such that, all else being equal, pharmaceuticals benefiting the former should be funded in preference to pharmaceuticals benefiting the latter, with the ultimate objective being the equalisation of the respective groups' health.

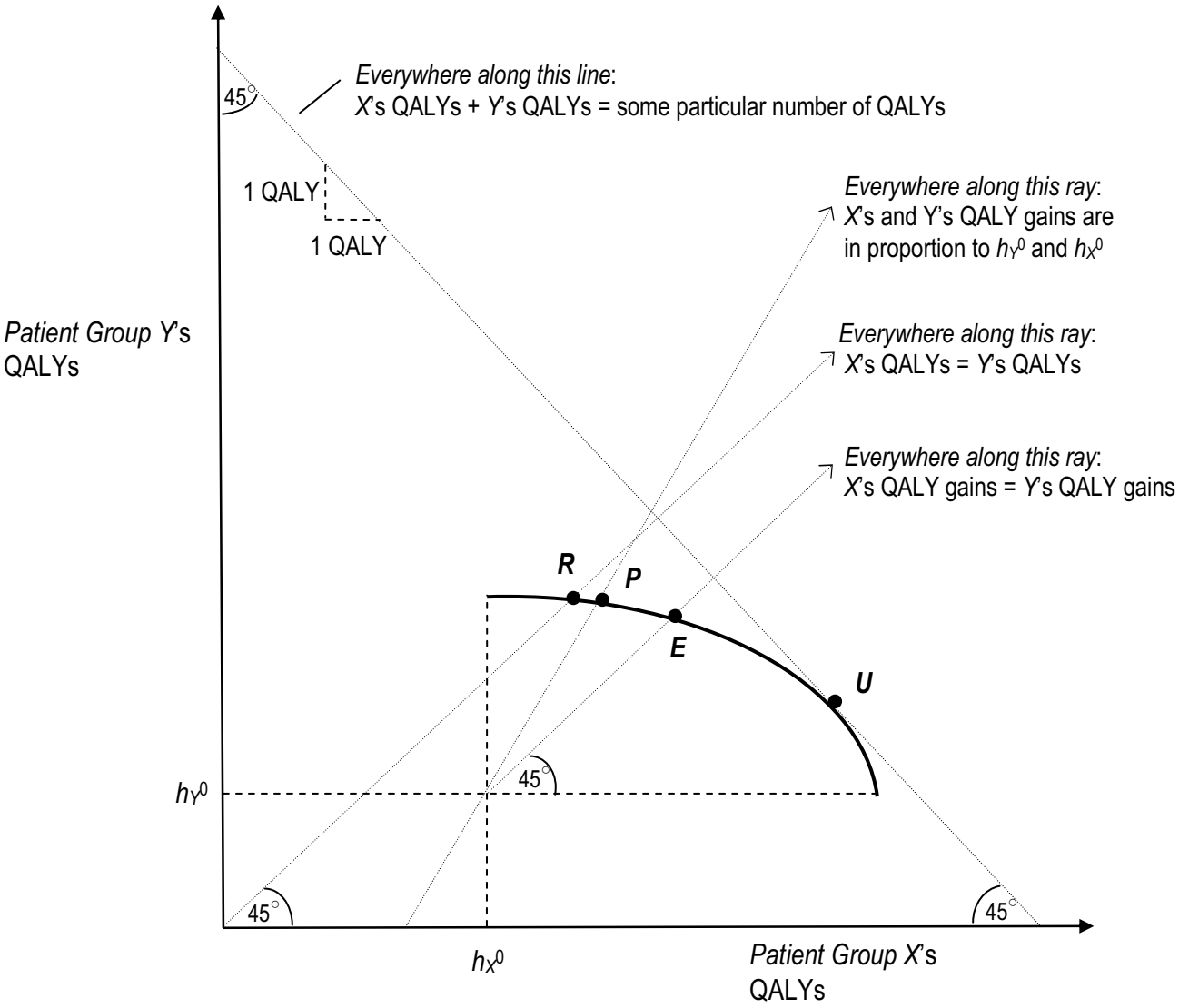
More generally, this value judgement corresponds to John Rawls'[15] well-known theory of justice, "Justice as Fairness", which he advanced as an alternative to utilitarianism (Rawls 1971). Central to the development of Rawls' theory is a famous 'thought experiment' in which people are asked to choose the moral principles they would like the society they inhabited to operate under. However, they have to make their choices from behind a 'veil of ignorance'; that is, in ignorance of their own particular characteristics, such as their wealth and natural abilities, in that society. Rawls agues that most people would prefer a world in which the well-being of the worst off in society was maximised (because they might turn out to be that person when the veil of ignorance is lifted). This is often known as the 'maximin' rule: the *maxi*misation of the *min*imum (or worst possible) outcomes.

[13] Jeremy Bentham (1748-1832) was an English philosopher and legal and social reformer. Bentham originally expressed this principle in terms of "the greatest happiness of the greatest number of people"; however, he later realised that it included two different and potentially conflicting principles (maximands), and so he abandoned the second part ("the greatest number of people"). Thanks to Martin Wilkinson for reminding me of this.

[14] This is sometimes known as an 'isovalue' or 'indifference' curve.

[15] John Rawls (1921-2002) was an American philosopher.

Figure 2: The 'best' allocation of QALYs under four (illustrative) value judgements: Utilitarianism (U), Rawlsianism (R), 'QALY gains in proportion to relative needs' (P) and 'Equal QALY gains to all patient groups' (E)



In the present context,[16] the Rawlsian value judgement translates, in essence, into an equal, or as equal as possible, allocation of health to patient groups. Again with reference to Figure 2, this is where *Patient Group X's QALYs = Patient Group Y's QALYs*, which occurs at all points along the 45˚ line (line of equality) out of the origin (slope = 1). (Clearly, this 45˚ line represents equality between the variables on the horizontal and vertical axes.)

The intersection of this 45˚ line with the health possibility (efficiency) frontier identifies the Rawlsian allocation of PHARMAC's budget (point *R*). Only at point *R* is the allocation of QALYs between the two patient groups both economically efficient (as discussed earlier) and equal (as well as being affordable to PHARMAC). Notice, in particular, that point *R* (the Rawlsian allocation) is very different to point *U* (the utilitarian allocation).

[16] As pointed out by Williams & Cookson (2000, pp. 1897-8), however, Rawls explicitly rejected the application of his theory of justice to the allocation of health.

Value judgement 3: 'QALY gains in proportion to relative needs'

This value judgement — requiring that QALY gains are distributed between patient groups in strict proportionality to their relative health needs (i.e. their respective QALYs if they were not to receive the pharmaceuticals) — was considered by PHARMAC as a possible decision criterion (see PHARMAC 2003, p. 5-6).

With reference to Figure 2, this corresponds to point *P* in Figure 2. The geometry of this allocation's derivation is quite complicated to explain. It suffices here to note that each patient group's QALY gain is determined in relation to the ratio h_Y^0/h_X^0 (i.e. their relative health needs). As can be seen in the figure, *Patient Group Y*, which is needier (i.e. $h_Y^0 < h_X^0$), gains more QALYs than *Patient Group X*.

As you would expect given their definitions, point *P* is closer to point *R* (the Rawlsian allocation) than point *U* (the utilitarian allocation).

Value judgement 4: 'Equal QALY gains to all patient groups'

This requires that the QALY *gains* to each patient group are the same (in contrast to Rawlsianism, for which the final number of QALYs that each group ends up with is the same). Unlike the three previous ones, this value judgement does not have a noteworthy pedigree (it is simply acknowledged in the sources cited earlier).

With reference to Figure 2, this value judgement requires that *Patient Group X's* QALY gain = *Patient Group Y's* QALY gain. As for the Rawlsian value judgement, such combinations are to be found along a 45˚ line (the 'line of equality'). But this time, instead of passing through the origin, this 45˚ line passes through the intersection point of the QALYs for each patient group if they were not to receive the pharmaceuticals (h_Y^0 and h_X^0). The intersection of this 45˚ line with the health possibility (efficiency) frontier (point *E*) identifies PHARMAC's budget allocation under the value judgement.

Maximum QALY gains versus their distribution

Overall, as can be seen in Figure 2, the four value judgements have very different implications for the total number of QALYs gained and their distribution and, most importantly in the present context, for the distribution of PHARMAC's budget between *Drug X* and *Drug Y* (the 'high cost' pharmaceutical).

The utilitarian value judgement (*U*) results, by definition, in the maximum total number of QALYs. The next greatest number of QALYs arises from the 'equal QALY gains to all patient groups' (*E*) value judgement, followed by 'QALY gains in proportion to need' value (*P*) and, last of all, the Rawlsian value judgement (*R*). This ranking can be confirmed easily in Figure 2 by laying three other lines ('isovalue curves') with slope = –1 (i.e. at 45˚ from both axes) through the *E*, *P* and *R* tangency points respectively. The further the line is from the origin, the greater the total number of QALYs.

This ranking ($U > E > P > R$) is reversed with respect to the extent to which QALY gains are delivered to *Patient Group Y* (with, as explained earlier, the greater need for treatment and lower capacity to benefit respectively): i.e. $R > P > E > U$.

Likewise, Rawlsianism (*R*) results in the greatest amount of spending on *Drug Y* ('the high cost pharmaceutical' in this simple model), followed by 'QALY gains in proportion to relative need' (*P*), then 'equal QALY gains to all patient groups' value judgement (*E*), and, lastly, utilitarianism (*U*).

The key idea revealed by this theoretical demonstration is that economics-based decision making requires consideration of the extent to which particular allocations of health (QALYs)

available from a given PHARMAC budget are preferred over other allocations. Decision makers need to consider the tradeoffs between the *maximum* QALY gains possible (the utilitarian value judgement, *U*) and the *distribution* of those gains (including, but not limited to, the three other value judgements considered above, *E*, *P* and *R*).

Equity weighting?

In theory, it is possible to represent *any* value judgement (i.e. ethical position or theory of social justice/equity) by attaching appropriate weights to the health (QALY) gains possible from PHARMAC's budget. With reference to Figure 2 again, for example, the Rawlsian value judgement could be reflected by attaching greater weight to *Patient Group Y*'s QALY gains (the needier group) than to *Patient Group X*'s. PHARMAC could then allocate its budget so as to maximise the sum of the *weighted* QALY gains.

But, of course, the obvious question is: What are the appropriate weights? Thus the development of 'equity weights' to reflect a range of social justice/equity concerns is a significant focus of contemporary health economics research (e.g. Nord 1995, Bleichrodt et al. 2004). However, notwithstanding these efforts, according to Williams & Cookson (2000) and Powers & McFaden (2000), valid and reliable methods for estimating such weights (particularly in the context of CUA and QALYs) that are capable of being used for practical (policy-based) decision making are currently under-developed, so that they are generally unavailable.

Williams & Cookson (p. 1901) summarise the literature thus:

> Generally-speaking, the work of economists in seeking greater quantification in this field falls into two classes: that which addresses equity-efficiency trade-offs in the distribution of health [in essence, equity weights] explicitly in a quantitative manner, but currently lacks the empirical data with which to support the assumed numerical relationship; and that which attempts to estimate such trade-offs empirically using questionnaire methods, but without having an explicit theory of justice into which to insert and interpret the findings. It is a rather unsatisfactory state of affairs.

Thus equity weighting is unlikely to represent a practical way forward in the foreseeable future. Other, more practical, considerations associated with possible improvements to PHARMAC's decision-making approach are considered in the following section.

3. Possible improvements to PHARMAC's approach to deciding which pharmaceuticals to fund, including high cost ones

(PHARMAC's questions pertaining to this section)

(d) Could cost-utility analysis be used more effectively when considering 'high cost' pharmaceuticals? If so, how?

(e) What, if any, changes do you recommend PHARMAC make to its current decision-making process for 'high cost' pharmaceuticals (and others)?

(f) What role should rule of rescue play in assessing High Cost Pharmaceuticals for funding?

(g) What are the arguments for and against paying a higher price (per QALY gained, for example) for pharmaceuticals for those who are worse off clinically with poor quality-adjusted life expectancy, but of arguably greater need (for example, the terminally ill)?

(h) Are there any general comments that you wish to make?

PHARMAC's current approach

According to its *Operating Policies and Procedures* (2001, p. 4-5), PHARMAC's current approach to deciding which pharmaceuticals to fund (including high cost ones) is as follows.

> PHARMAC uses the criteria set out in this clause, where applicable and giving such weight to each criterion as PHARMAC considers appropriate, to make decisions about proposed changes to the Schedule [i.e. which pharmaceuticals to fund]. ... These criteria are:
>
> (a) the health needs of all eligible (PHARMAC's footnote: as defined by the Government's then current rules of eligibility) people within New Zealand;
>
> (b) the particular health needs of Maori and Pacific peoples;
>
> (c) the availability and suitability of existing medicines, therapeutic medical devices and related products and related things;
>
> (d) the cost-effectiveness of meeting health needs by funding pharmaceuticals rather than using other publicly funded health and disability support services;
>
> (f) the budgetary impact (in terms of the pharmaceuticals budget and the Government's overall health budget) of any changes to the Schedule;
>
> (g) the direct cost to health service users;
>
> (h) the Government's priorities for health funding, as set out in any objectives notified by the Crown to PHARMAC, or in PHARMAC's Funding Agreement, or elsewhere; and
>
> (i) such other criteria as PHARMAC thinks fit. PHARMAC will carry out appropriate consultation when it intends to take any such "other criteria" into account.

But what do the criteria mean?

As they are written here, these criteria (value judgements) are imprecise (for PHARMAC's stated purpose of "mak[ing] decisions about proposed changes to the Schedule"). For example, how is "(a) the health needs of all eligible people within New Zealand" to be interpreted as a *decision* criterion? Does it mean that the sickest patient groups are to be favoured over other groups? How is "(b) the particular health needs of Maori and Pacific

peoples" different from criterion (a)? (Depending on how (a) and (b) are interpreted, then isn't there the possibility of double counting?) And so on.

Overall, PHARMAC's criteria seem less precise than NICE's (2005) guidelines for the UK's National Health Service (NHS) or, as summarised by PHARMAC (2003, p. 5), Australia's criteria for funding its Life Saving Drug Programme. (Though, this is not to say that NICE's or Australia's guidelines/criteria are necessarily correct.)

With respect to PHARMAC's stated procedure of "giving such weight to each criterion as PHARMAC considers appropriate" (as above), how does PHARMAC determine these weights? In particular, how important is cost per QALY gained relative to the other criteria? According to Metcalfe et al. (2003, p. 1 of 10): "The PHARMAC Board uses [the above] decision criteria each time it makes a decision. Cost-effectiveness is just one of these criteria." But how important is it relative to the others?

Ironically, when reviewing the Ministry of Health's Special High Cost Treatment Pool (SHCTP), PHARMAC (2003, p. 4) complains: "It is very difficult to ascertain how exactly the [SHCTP cost-effectiveness] evaluation is done and we understand that even if a [CUA] is completed it is not clear how much weighting it is given. Often if an application is declined it is because there is very little clinical information."

In general, there is a tendency in decision-making situations like this for criteria that are based on so-called 'hard' data, such as cost per QALY estimates, to 'overwhelm' other criteria based on less precise definitions or 'soft' data, such as PHARMAC's other criteria above. Such decision making often ends up being based mainly on the hard data criteria, with the other criteria, to a greater or lesser extent, in effect being paid 'lip service'.

This tendency towards (unintentionally) biased decision making can be reduced by specifying the decision criteria, and their relative importance, as precisely as possible — even for criteria that are qualitative in nature — and also ensuring the over-arching decision-making approach is as explicit and transparent as possible. The remainder of the review considers ways by which PHARMAC might do this.

A possible four-step approach

The following 'four-step approach' *could* form the basis for an explicit declaration of PHARMAC's over-arching approach. NICE (2005) recommends a similar method in its NHS prioritisation "guidelines". (Please note, I am not recommending that PHARMAC necessarily follows this approach; rather I am recommending that PHARMAC *considers* something like this.)

I am not implying that PHARMAC does not already *think* in the way represented below. Rather, it is that, as represented in its *Operating Policies and Procedures* (reproduced above) and related literature (e.g. PHARMAC 2004, Metcalfe 2003), PHARMAC is not explicit about its approach.

Implicit in the following discussion (as elsewhere in this review) is the assumption that PHARMAC has flexibility in allocating its pharmaceuticals budget. That is, it has either new funding to allocate or it is engaging in a PBMA-type exercise.[17]

[17] In essence, PBMA ('Programme Budgeting Marginal Analysis'; Mooney 1994, Scott et al. 1999) involves asking decision makers two fiscally-neutral questions: "If you had an additional $*N* available in your budget, what pharmaceuticals would you spend it on?"; and "If you had to cut $*N* from your existing budget, what pharmaceuticals would you not fund?" PBMA has been trialled in the United Kingdom and Australia, and in New Zealand; for a review of these experiences and references see Ashton et al. (1999).

Originally proposed by Hope et al. (2002) for prioritising health care spending in general, and used by Oxfordshire Health Authority (UK), the following four steps are re-phrased here in terms of deciding which pharmaceuticals to fund.

1. For a pharmaceutical that is being considered for funding, estimate its cost per QALY (gained).

2. Compare this estimate with the maximum cost per QALY — i.e. a threshold/cut-off cost — that PHARMAC is prepared to pay regardless of the pharmaceutical's 'performance' on the other decision criteria (addressed below).[18]

3. If the pharmaceutical's cost per QALY is less than the cut-off cost, then the presumption is in favour of funding the pharmaceutical, and a special reason would be required not to do so. If the pharmaceutical's cost per QALY is more than cut-off cost, then the presumption is that it should not be funded, and a decision to do so would depend on the following consideration (step).

4. If the pharmaceutical's cost per QALY is more than the cut-off cost, then two questions arise. First, based on PHARMAC's other decision criteria, is paying some amount more than the cut-off cost per QALY justified for the pharmaceutical? And if it is, then is it worth paying the required amount more? (In other words, does paying more represent good value for money?)

According to Hope et al. (p. 147):

> This approach is flexible. It ensures that the decision-making group [i.e. PHARMAC, in the present context] takes into account the key factors of cost and effectiveness, while also allowing other values to be considered. The group can recommend [pharmaceuticals] that would not be supported by the QALY calculation if it believes that there are further grounds — for example, those espoused by needs theories [e.g. Rawlsianism, etc., as in the previous section] — that justify straying from the QALY calculation. This approach requires the group to make a judgement as to how much weight should be given to these further grounds in terms of how much more than the [cut-off cost] is justified.

More specifically, Step 4 can be thought of in the following way. Suppose that a pharmaceutical with a cost of $50,000 per QALY were being considered for funding and PHARMAC's cut-off cost were $10,000 per QALY. Therefore each QALY derived from a given amount of spending on the high cost pharmaceutical has an opportunity cost of five QALYs that could be gained from other (lower cost) pharmaceutical spending ('on average'). Hence, in this case, the question for decision makers is: Based on PHARMAC's other decision criteria, are the QALYs from the high cost patient group worth (at least) five times more than the QALYs from other patient groups? If so, then the high cost pharmaceutical represents good value for money and should be funded. If not, then the high cost pharmaceutical should not be funded.

The suggestion (step 2) that PHARMAC specify a maximum cost per QALY that it is prepared to pay, regardless of the pharmaceutical's 'performance' on the other decision criteria, is intended to reduce PHARMAC's decision-making costs in terms of the time and effort expended. This cut-off cost should be set low enough that there would be only a very small chance that, were PHARMAC's other decision criteria to have been considered, then a pharmaceutical that passed this 'test' (was below the cut-off cost) would be displaced by a higher cost pharmaceutical (above the cut-off cost).

Although not mentioned in the four steps, another cut-off cost per QALY could also be set (again to reduce PHARMAC's decision-making costs) — but this time a *minimum* cost per

[18] For example, this maximum might be (or not) around the $10,000 per QALY gained of most Pharmaceutical Schedule listings (PHARMAC 2003).

QALY that PHARMAC would '*never*' be prepared to pay (e.g. $500,000 per QALY), regardless of a pharmaceutical's (mitigating) performance on the other decision criteria.

Thus PHARMAC's decision-making efforts, in terms of considering criteria other than just cost per QALY, would be concentrated on pharmaceuticals with a cost per QALY between these two cut-offs. Pharmaceuticals outside this range would receive only a perfunctory form of analysis, as the likely benefits of greater analytical precision would be small. Performing different intensities of analysis is consistent with PHARMAC's four levels of economic analysis with respect to the time and effort it devotes to particular pharmaceuticals (Metcalfe et al. 2003, Table 2).

Some common ethical issues (value judgements)

After explaining the 'four-step approach' (as above), Hope et al. (2002, pp. 147-51) go on to consider the following ethical issues "that arise in practice and that raise the question of whether more (or less) should be spent per QALY than the [cut-off cost]." NICE (2005, p. 6) recommends similar "social value judgements that are involved in developing conclusions about cost effectiveness and particularly those with implications for priority setting, resource allocation and so-called rationing."

This list of possible value judgements is not exhaustive; for a more comprehensive survey, see Schwappach (2002) for example. The main purpose of reproducing them here (and discussing them briefly) is to illustrate how such value judgements can be articulated relatively clearly and simply — in contrast to the imprecision of PHARMAC's decision criteria (reproduced earlier). (As in Section 1 of this review, I am not advocating for any of them.)

"Should treatments for the young have a greater priority than treatments for the old?"

The value judgement implied by answering this question in the affirmative is that the younger a person is, the less of a 'fair innings' she has enjoyed with respect to the natural human life span. Therefore a young person's QALYs gained should be worth more than the same number of QALYs to an older person, all else being equal (Williams 1997).

On the other hand, "[t]he main criticism that has been levelled against the fair innings argument is that this strong form of 'ageism' is incompatible with the duty of care that a civilised society owes to its elderly population." (Williams & Cookson 2000, p. 1876).

NICE (2005) recommends that "health should not be valued more highly in some age groups than others." (Recommendation 6, p. 3).

"Should identifiable patients be favoured over nonidentifiable patients? The rule of rescue."

The value judgement implied by this question's answer in the affirmative is that the QALYs gained from preventing the immediate premature death of, in the extreme, a single identifiable individual (e.g. a heart attack sufferer) are inherently more important (valuable) than the same number of QALYs gained from slightly improving the health (or reducing the risk of ill health) of a group of people.

Hadorn (1991, pp. 2219) considers the 'rule of rescue' to be intrinsic to human nature: "people cannot stand idly by when an identified person's life is visibly threatened if effective rescue measures are available." Notwithstanding its naturalness, however, like the other value judgements considered here, there are limits to how far the rule ought to be accommodated. "Clearly … there is also an emotional component to the rule of rescue that can interfere with the development and implementation of fair allocation systems. Society

cannot afford to provide every possible beneficial service [or pharmaceutical] to every patient — identified or not ..." (p. 2219).[19]

Similarly, according to Williams & Cookson (2000, p. 1894):

> However, it is hard to give a rational justification for the rule of rescue insofar as it conflicts with other principles of justice. It seems irrational to devote resources to people who happen to be in immediate distress if it is true that other people with a greater claim on those resources (e.g. those who need them more, or who are more disadvantaged) will lose out as a result. In defence of the rule of rescue in such cases, however, it can be replied that some ethical rules are such deeply embedded social conventions that they should be followed unless the consequences of doing so are clearly disastrous.

NICE recommends that "considerable care should be taken when applying the 'rule of rescue' (Recommendation 13, p. 4).

"Should palliative care be given higher priority than would result from the QALY calculation?"

The value judgement implied here is that the final stage of a person's life, as requiring palliative care, is intrinsically of greater importance (value) than other stages (of equal QALYs), both for the individual herself and her friends and family. Therefore QALYs gained via palliative care should be worth more than the same number of QALYs gained via other types of treatment, all else being equal.

"Should higher priority be given to those who are particularly badly off with regard to their health?"

This is qualitatively the same as the Rawlsian and 'QALY gains in proportion to relative needs' value judgements demonstrated in the previous section. Arguably, PHARMAC's criteria (a) and (b) could be interpreted as having such a meaning.

Surprisingly, given its discussion of egalitarianism, NICE (2005) does not make any recommendations related to relative 'needs'.

"Should higher priority be given if there is no alternative treatment?"

According to this implied value judgement, patient groups for which there is no alternative treatment to the pharmaceutical being considered for funding should be favoured over other groups for which alternative treatments are available, all else being equal. Arguably, PHARMAC's criterion (c) could be interpreted to mean this.

NICE recommends that: "Special consideration should be given to innovations that provide significant improvements in health for previously untreatable conditions (i.e. beyond 'best supportive care'), but taking account of the prognosis, the magnitude of the gain in health, and the cost." (Recommendation 15, pp. 4-5)

"How is 'double jeopardy' to be dealt with?"

'Double jeopardy' relates to the possibility of co-morbidities in the patient group being considered for a pharmaceutical, that would have the effect of lowering the QALYs gained from using the pharmaceutical, all else being equal.

[19] In his commentary for PHARMAC, Hadorn discusses the rule of rescue in detail and concludes (p. 5): "We must, however, recognize the our 'prudential tendencies' to rescue the doomed are often irrational and must not be used as a basis for health policy."

One solution to this problem (if indeed it is a 'problem', rather than being just a 'fact of life' — itself a value judgement!) is to effectively ignore the co-morbidities by estimating the QALYs that would be gained for the population as a whole (i.e. without the co-morbidities).

NICE acknowledges that 'double jeopardy' is an issue but is unsure how it "might best be incorporated" (p. 24).

Whose value judgements?

The six value judgements surveyed above, and the other examples in the previous section, are a subset of possible value judgements. Notwithstanding the infinite number of value judgements that are available in theory, to a greater or lesser extents individually, these are likely to command fairly popular support. As noted earlier, a more comprehensive survey of common value judgements is available from Schwappach (2002).

As PHARMAC has clearly done so to some extent already, determining what the relevant criteria are for deciding which pharmaceuticals to fund should be done by consulting PHARMAC's constituencies (or stake-holders to the decision), including tax-payers, patients, health care providers, the Government, etc. Such a consultation process can be informal or formal (as in the example below) in nature.

Also, given the continually changing socio-demographic characteristics of New Zealand's population (e.g. due to immigration, and also the 'ageing population'), it is possible that these criteria and their relative importance (discussed below) will change over time. Therefore the process by which PHARMAC determines them may need to be flexible. This need for flexibility is strengthened by the inevitably concomitant changes in pharmaceuticals that are available and (if recent history is anything to go by) the future institutional reforms to New Zealand's health system.

An example of a formal consultation process is NICE's establishment of a Citizens' Council, comprising 30 members of the general public from England and Wales, "to help develop the broad social values that NICE should adopt in preparing its guidance." (NICE 2005, p. 8). The (implied) raison d'être of the Council is as follows (NICE 2005, p. 8):

> While the members of NICE's advisory bodies are appointed for their competence in making scientific value judgements neither they, nor the [Institute's] board, have any legitimacy to impose their own social value judgements on the NHS and the patients that the service seeks to serve.

NICE has also conducted at least one telephone survey of the UK general public that asked questions related to these issues.

Please note, I am not recommending that a formal consultation process like a Citizen's Council necessarily be established by PHARMAC. There are obvious and significant costs associated with the creation and support of such institutions, and further research would be required to establish that they are worthwhile having (personally, I doubt it).

What are the relative weights?

Having specified the criteria for deciding which pharmaceuticals to fund (clearly, no easy task!), an important issue remains: What are their relative importance (weights) relative to each other? In other words, how is a pharmaceutical's cost per QALY and its 'performance' on the other decision criteria to be traded-off against each other and ultimately aggregated in order to decide whether to fund the pharmaceutical or not?

For example, suppose a given pharmaceutical costs $60,000 per QALY gained (i.e. six times the $10,000 per QALY gained of most Pharmaceutical Schedule listings; PHARMAC 2003).

But suppose that — here adapting PHARMAC's (2001) and Hope et al.'s (2002) (implied) value judgements above — its patient group is young, Maori, and would die without the pharmaceutical (rule of rescue), and no alternative treatments are available. How is the pharmaceutical's conflicting 'performance' on these two groups of possible criteria (and any others that might matter) to be aggregated in order to reach a decision as to whether or not to fund the pharmaceutical?

Three types of solution to this 'aggregation problem' exist, as distinguished by how explicit and transparent they make the tradeoffs between the criteria.

Implicit weighting

The least explicit and least transparent type of solution is for the tradeoffs between criteria to be evaluated in an essentially implicit and non-transparent (opaque) fashion, on a case-by-case basis. This appears to be PHARMAC's approach at present.

According to its *Operating Policies and Procedures* (PHARMAC 2001, p. 10), when considering a pharmaceutical for funding, "PHARMAC will consult when it considers appropriate with any section of the public, groups, or individuals that, in the view of PHARMAC, may be affected by its proposals (which may, according to the circumstances, include suppliers, PTAC [The Pharmacology and Therapeutics Advisory Committee], health professionals, community or patient groups, Maori and Pacific peoples and other groups)."

Notwithstanding the imprecision of PHARMAC's criteria (reproduced earlier), tradeoffs between them are to be resolved and the criteria ultimately aggregated by "giving such weight to each criterion as PHARMAC considers appropriate" (as noted earlier). As far as I can discover, these weights, or any other measure of the relative importance of the decision criteria, are not publicised.

Also, PHARMAC does not appear to be explicit in *explaining* its funding decisions. According to its *Operating Policies and Procedures*, having made its decision, "PHARMAC will, when it considers it appropriate to do so, take measures to *inform* the public, groups and individuals of PHARMAC's decisions concerning the Schedule." (p. 11; my italics). Notice, in particular, that PHARMAC does not undertake to explain the reasons for its decision (PHARMAC may in fact do so nonetheless).

In contrast, NICE recommends that its "guidance should *explain, explicitly, reasons* for recommending — as cost effective — those interventions with an incremental cost-effectiveness ratio of £20,000 to £30,000 per QALY." (NICE 2005, Recommendation 5, p. 3; my italics).

Equity weighting of QALYs

As discussed in the previous section, equity weighting has been proposed, in the context of CUA and QALYs, as a general method for formalising a range of ethical and social justice/equity criteria. However, as noted also in the previous section, valid and reliable methods for estimating such weights are currently unavailable.

Thus, "NICE does not include equity weighting in its approach to cost-utility analysis (NICE 2004)." (NICE 2005, p. 19). I would suggest that PHARMAC does not either.

Nonetheless, as discussed earlier, such weights can be considered *implicitly* when Step 4 of the above-mentioned 'four-step approach' is being applied. As illustrated via the hypothetical example of a pharmaceutical costing $60,000 per QALY relative to a cut-off of $10,000, such weights are effectively the ratio of the high cost pharmaceutical's cost per QALY to the cut-off cost — a weight of '6' in this example. Thus *implicit* equity weights can be calculated, and the

question asked: Are they acceptable (i.e. not too high) to decision makers with respect to favouring this particular patient group over others?

Multiple Criteria Decision Making

The third and final type of solution considered here to the problem of how to determine the relative importance of the criteria for deciding which pharmaceuticals to fund is Multiple Criteria Decision Analysis (MCDA). It is discussed in greater detail than the two other solutions above because I believe PHARMAC should *consider* using it.[20]

MCDA consists, in the present context, of *formal*, and often *practical*, methods for determining explicit tradeoffs between decision criteria and aggregating them to help decide which pharmaceuticals to fund. Such methods are most commonly implemented via additive 'points systems' (also known variously as 'scoring', 'point-count' and 'linear' systems/models/tools, or more formally in the academic MCDA literature as 'additive multiple criteria value models with ordered categorical scales').

Additive points systems (hereinafter simply 'points systems') are a common solution to the pervasive problem in a wide variety of applications (surveyed below) of how to combine alternatives' characteristics on multiple criteria (or attributes) for the purpose of obtaining an overall ranking of alternatives.

An example of a hypothetical points system and the resulting ranking of four imaginary pharmaceuticals ('Drug A' to 'Drug D') produced from it appears in Figure 3 below. Thus a points system is a schedule of 'point values' for each criterion, where each criterion is demarcated into two or more mutually-exclusive levels and the point values represent the relative importance of the criteria and levels for the decision at hand (which pharmaceuticals to fund). A points system's criteria may be quantitative or qualitative in nature, and for criteria that are not naturally categorical it is usually possible to represent them in terms of levels that are appropriate to the application (which must be listed within each criterion from lowest ranked to highest ranked).

A points system works by each alternative being considered being graded on the system's criteria and the corresponding point values *summed* to obtain a total score for each alternative, by which they are ranked or classified relative to each other (where the total scores have no meaning other than for determining this ranking or classification). (See the ranking of the four imaginary pharmaceuticals at the bottom of Figure 3.)

Provided the decision criteria and their point values are valid, the ranking produced by a points system can be used as a *guide* as to (in the present context) which pharmaceuticals to fund (and which not to). It should be emphasised that points systems are *not* a replacement for 'human' decision making. Rather they are a *tool* to support decision makers by increasing the validity and reliability of the processes employed, and to make them more explicit and transparent (i.e. accountable). Points systems can also speed up the decision making process, by providing a 'first cut' at prioritising the pharmaceuticals being considered for funding. Ultimately, of course, the final decision of which pharmaceutical to fund rests with decision makers.

It is also worthwhile emphasising that MCDA, as implemented via points systems — which hails from the field of Operations Research (including Conjoint Analysis) — is outside the traditional Health Economics paradigm of attaching equity weights to QALY estimates for CUA (as mentioned above). In particular, the tradeoffs are between decision criteria rather than different groups' QALYs. Thus points systems are not the same, conceptually, as equity

[20] This section of the review received the most attention (mostly favourable, but also unfavourable) from the ten commentators.

weights applied to QALYs (which, as discussed above, at a practical level are currently unavailable).

The notion of using points systems as a tool for assisting PHARMAC to decide which pharmaceutical to fund is not exceptional as points systems are widely used in New Zealand and internationally for three types of health care decision making (based on the subjective judgements of decision makers — as is PHARMAC's decision making). They are: (1) prioritising publicly-financed spending across different health services (*horizontal* priority setting — analogous to PHARMAC's pharmaceutical funding decisions), (2) prioritising patients for access to a given service (*vertical* priority setting), and (3) assessing students for admission to educational institutions (e.g. medical, dental and pharmacy schools) and health care professionals for job openings (e.g. junior doctors for hospital posts).[21]

With respect to type (1) above, Ryan et al.'s (2001) systematic review of methods for eliciting health care preferences identified eight examples of points systems used by district health authorities and hospitals in Scotland and England to prioritise health spending proposals (e.g. Farrar et al. 2000, Ham 1993) and for prioritising Medicaid coverage in the US state of Oregon (Oregon Health Services Commission 1991).

With respect to type (2), New Zealand and Canada, in particular, have developed points systems at a health system-wide level over the last decade for prioritising access to a wide range of elective health services. New Zealand has points systems for cardiology and cardiac surgery (five types), gynaecological and infertility treatments, spinal surgery, cataract surgery, vascular surgery, hip and knee replacements, and paediatric surgery (Ministry of Health 2006), and Canada has points systems for cataract surgery, general surgical procedures, hip and knee replacement, magnetic resonance imaging scanning, and children's mental health (Noseworthy et al. 2003). Points systems have also been used in the United Kingdom and their system-wide adoption debated (Edwards 1999, Derrett et al. 2002). For a survey of these three countries' points systems, including a literature review, see MacCormick et al. (2003).

Finally, with respect to type (3), points systems are also widely used internationally for assessing students for admission to educational institutions, such as medical, dental and pharmacy schools (e.g. Parry et al. 2006, Latif 2004, Collins et al. 1995, Van Susteren et al. 1999), and similar systems are used for selecting health care professionals for job openings, such as junior doctors for hospital posts (e.g. Walzman et al. 2005).

Points systems are used in other 'non-health' areas too. For example, New Zealand (and other countries) uses a points system for assessing immigration applicants (Immigration New Zealand 2005). Points systems are also widely used for assessing job applicants in general and employees' performances (e.g. Barclay 2001, Blackham & Smith 1989), and other, less well-known, applications, as surveyed by Hastie & Dawes (2001), include predicting parole violations, business bankruptcies and college graduations.

The popularity of points systems, in general, derives from their having been near-universally found (in a large number of studies of a wide variety of applications, including non-health ones) to out-perform purely intuitive decision-making approaches. Specifically, points

[21] Points systems are also used for diagnosing patients and predicting health outcomes. The Medical Algorithms Project (Svirbely & Iyengar 2005) documents more than 8,700 algorithms from 40 branches of medicine, of which a large proportion are points systems. In contrast to the three types of application mentioned above, however, which are all based on the subjective judgements of decision makers, most points systems for diagnosis/prediction purposes are based on statistical analyses of the medical and epidemiological relationships between the outcomes of interest and their determinants.

systems have been found to be more accurate for decision making and prediction than the unaided 'expert' judgements of decision makers (as well, points systems are simple to use).[22]

This is the near-universal finding of a large number of studies, as surveyed by Hastie & Dawes (2001), who concluded that (p. 53):

> [Points systems are] surprisingly successful in many applications. We say surprisingly because many judges claim that their mental processes are much more complex than the linear summary equations would suggest but empirically the equation does a remarkably good job of 'capturing' their judgment habits.

This is because (p. 62):

> The mind is in many essential respects a linear weighting and adding device. In fact much of what we know about the neural networks in the physical brain suggests that a natural computation for such a 'machine' is weighting and adding, exactly the fundamental processes that are well described by linear equations.

Similarly, according to Hadorn et al. (2003, p. 49):

> [Points systems] are, as a rule, more accurate than human predictors. This is not surprising, as it has been known for decades that human cognitive performance is limited with respect to complex multi-variable judgment and prediction tasks (Meehl 1954).

In all types of application, having specified a given points system's criteria and the levels within each criterion,[23] the task facing the decision maker (or a group of decision makers) is to determine the point values (sometimes known as 'scoring' the system) so that her/his (their) preferred ranking of alternatives is reproduced by the ranking of total scores.

Ryan et al.'s (2001) systematic review identified three methods to have been used for determining the point values for points systems for horizontal (health care) priority setting: (1) the 'allocation of points' method, whereby decision makers allocate a fixed number ('budget') of points (e.g. 100) amongst the criteria which are interpreted as criterion weights (e.g. Oregon Health Services Commission 1991); (2) full-profile conjoint analysis involving (discrete) pairwise choices (e.g. Farrar et al. 2000); and (3) the essentially ad hoc assignment of point values by decision makers (e.g. Ham 1993).

Of these three scoring methods, Ryan et al. concluded (in the context of health care priority setting) that conjoint analysis involving pairwise choices is the best with respect to the usual methodological assessment criteria of validity, reproducibility (reliability), internal consistency, acceptability to respondents (decision makers) and cost.

A particular strength — of choice-based scoring methods in general (including the 1000Minds software/method, as discussed below) — is that the decision maker is required to confront explicit trade-offs and make choices, and in a way that most people find acceptable given that everyone has experience at choosing things. "The advantage of choice-based methods is that choosing, unlike scaling, is a natural human task at which we all have considerable experience, and furthermore it is observable and verifiable." (Drummond et al. 1997, p. 148).

In addition to the methods identified by Ryan et al., New Zealand's and Canada's points systems were scored using group consensus methods (e.g. the Delphi technique) and rating scale-based conjoint analysis involving regressions of visual analogue scale ratings of clinicians' assessments of patients' urgency against their characteristics with respect to the

[22] This accuracy is despite points systems ruling out interaction effects between criteria by their construction — i.e. a points system's criteria are independent, as they are combined additively.

[23] See Belton & Stewart (2002, pp. 55-59), for example, for a discussion of the issues to consider when identifying the criteria and levels.

points system's criteria (Noseworthy et al. 2003, MacCormick et al. 2003). However, the validity of the patient rankings (and the resulting health outcomes) produced by some of New Zealand's original points systems has been seriously criticised (e.g. Jackson et al. 1999, Seddon et al. 1999), casting doubt on the validity of the scoring method.

Finally, a choice-based method for determining a points system's points values similar to conjoint analysis (as mentioned above) that I am very familiar with, because I developed it with Franz Ombler,[24] is the method implemented as software known as 1000Minds (formerly *Point Wizard*) (Hansen & Ombler 2005a,b), that since late 2005 PHARMAC has had access to on a trial basis.

This method/software includes a fully-integrated process for multiple decision makers to work together as group. It is centrally administered and managed via the Internet and based on these six steps (performable as a group, remotely via the Internet): (1) Drafting the criteria for the ranking decision being considered; (2) Pre-testing the drafted criteria and levels; (3) Determining the relative importance of the criteria (their point values or weights); (4) Checking the 'face' validity of the new system before using it; (5) Implementing the system; and (6) Reviewing and fine-tuning the system (if and as desired).

Since 2004, 1000Minds has been used by the Ministry of Health in collaboration with groups of clinicians and their professional bodies to create points systems for prioritising patients for cardiac surgery, hip and knee replacements, vascular surgery, cataract surgery and infertility and gynaecological treatments respectively. An overview of this work appears in Appendix B.

Other software for performing MCDA exist too, such as *Expert Choice* (Expert Choice 2005). For a survey of Decision Analysis software in general (much of which is not relevant to PHARMAC's decision making, however, as they are for fundamentally different types of application), see INFORMS (2004).

If PHARMAC were interested in formalising its methods for determining explicit tradeoffs between decision criteria and aggregating them to help decide which pharmaceuticals to fund, then it might consider using software such as 1000Minds, *Expert Choice* or others. [Please note, I am not recommending that PHARMAC necessarily uses Multiple Criteria Decision Analysis or any of these software; rather I am recommending that it *considers* using them. In my opinion, further research (and experimentation) into the usefulness of these methods for PHARMAC would be worthwhile.]

[24] I am declaring an interest here.

Figure 3: A hypothetical (illustrative) points system for deciding which pharmaceuticals to fund and a ranking of four imaginary pharmaceuticals ('Drug A' to 'Drug D') produced from it

Cost per QALY	Points	Score
over $250,000	0	
$200,001 to $250,000	48	
$150,000 to $200,000	59	
$100,001 to $150,000	63	
$50,001 to $100,000	66	
$20,001 to $50,000	67	
$10,000 to $20,000	81	
below $10,000	127	
Relative need of patient group (health status if untreated)		
Low need (relatively good health status if untreated)	0	
Medium need (average health status if untreated)	45	
High need (relatively poor health status if untreated)	65	
Predominant ethnicity of patient group		
Non-Maori and non-Pacific Islands Peoples	0	
Maori or Pacific Islands Peoples	13	
Predominant age group of patient group		
Over 71 years	0	
31 to 70 years	21	
10 to 30 years	44	
Under 10 years	60	
Rule of rescue considerations		
If untreated, illness will NOT likely lead to death or serious disability	0	
If untreated, illness will likely lead to serious disability	10	
If untreated, illness will likely lead to death	22	
Effect on overall pharmaceuticals budget (likely "total spend")		
Relatively large "total spend"	0	
Medium "total spend"	2	
Relatively small "total spend"	64	
	Total Score:	

	Criteria							
Name (click to open)	Cost per QALY	Relative need of patient group (health status if untreated)	Predominant ethnicity of patient group	Predominant age group of patient group	Rule of rescue considerations	Effect on overall pharmaceuticals budget (likely "total spend")	Score	Rank
Drug D for consideration	$10,000 to $20,000	Medium need (average health status if untreated)	Non-Maori and non-Pacific Islands Peoples	Over 71 years	If untreated, illness will NOT likely lead to death or serious disability	Relatively small "total spend"	190	1st
Drug A for consideration	$20,001 to $50,000	High need (relatively poor health status if untreated)	Maori or Pacific Islands Peoples	31 to 70 years	If untreated, illness will likely lead to serious disability	Medium "total spend"	178	2nd
Drug B for consideration	$100,001 to $150,000	Medium need (average health status if untreated)	Maori or Pacific Islands Peoples	10 to 30 years	If untreated, illness will likely lead to serious disability	Relatively large "total spend"	175	3rd
Drug C for consideration	over $250,000	Low need (relatively good health status if untreated)	Non-Maori and non-Pacific Islands Peoples	Under 10 years	If untreated, illness will likely lead to death	Relatively small "total spend"	146	4th

4. Conclusion and Recommendations

As it should be in my opinion, PHARMAC's over-arching approach to deciding which pharmaceuticals to fund is *economics*-based, and heavily informed by cost-utility analysis (CUA). The central theme of this review is that the objective of *economics*-based decision making in this respect is the maximisation of 'value for money' from PHARMAC's budget. Such an approach does not prescribe particular value judgements (ethical positions) to the definition of 'value for money', but instead serves as an analytical framework.

CUA is an important input to this analytical framework, and it ought to be value judgement free. In particular, the utilitarian value judgement ought not to be automatically imposed. CUA's proper role is to provide information about the costs per QALY (gained) for different pharmaceuticals and patient groups. How PHARMAC uses this information for deciding which pharmaceuticals to fund, including high cost ones, is a separate matter that depends on the particular value judgements (ethical positions) concerning value for money that are adopted. Such value judgements are inevitable, and there is a wide range to choose from.

In my opinion, the challenge (not to be under-estimated) facing PHARMAC's decision makers is to better represent their preferred value judgements (and those of PHARMAC's constituencies) by clearly articulating valid criteria, and their relative importance, for deciding which pharmaceuticals to fund (including high cost ones). Currently PHARMAC appears to rely on 'intuitive-style' decision making, as characterised by decision criteria that are relatively imprecisely specified and weights that are implicit (and unpublicised). Accordingly, the following three recommendations are offered here to PHARMAC.

Recommendations

1. That PHARMAC be more explicit and transparent about its over-arching approach to deciding which pharmaceuticals to fund. The 'four-step approach' outlined in the previous section could form the basis for such a declaration.

2. That PHARMAC considers 'tightening up' how it expresses its decision criteria, at least as they are written in its *Operating Policies and Procedures*. (PHARMAC may want also to consult more fully its constituencies on what these criteria should be.)

3. That PHARMAC considers whether it wants to continue determining the relative importance of its decision criteria in an 'implicit' (intuitive) fashion or, alternatively, uses more formal methods available from the field of Multiple Criteria Decision Analysis (explained in the previous section). In my opinion, further research (and experimentation) into the usefulness of these methods for PHARMAC would be worthwhile.

Appendix A: Derivation of the 'health possibilities (efficiency) frontier', as used in Figures 1 and 2[25]

Before we begin, it should be emphasised that, like all models, the following representations are highly simplified and stylised abstractions of reality. Their purpose is to represent the fundamental theoretical aspects of a range of key concepts and relationships.

There are, of course, many types of pharmaceuticals, but for the sake of building up a simple two dimensional graphical model (as used in Figures 1 and 2 in the main text) we assume there are just two that PHARMAC is considering funding:[26] *Drug X* and *Drug Y*. The underlying model is built up from the following three components.

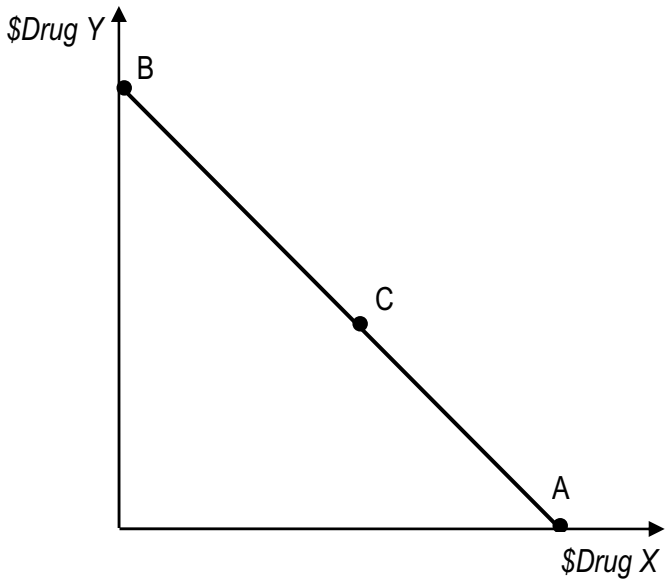
1. PHARMAC's budget constraint

We start with the obvious point that, given the prices for *Drug X* and *Drug Y* — denoted by p_X and p_Y — then PHARMAC's budget must be divided between spending on *Drug X* (i.e. *Drug X* $\times$ p_X = *$Drug X*) and spending on *Drug Y* (*Drug Y* $\times$ p_Y = *$Drug Y*); i.e. *$Drug X* + *$Drug Y* = PHARMAC's budget.

Thus PHARMAC's 'budget constraint' is illustrated in Figure A1, where the axes are *$Drug X* and *$Drug Y* (spending on each pharmaceutical) and the diagonal line (the 'budget line', with slope = –1) shows all affordable allocations of PHARMAC's budget between the two pharmaceuticals.

For example, at one extreme, all of PHARMAC's budget could be spent on *Drug X* and none on *Drug Y* (point A); or vice versa (point B); or half and half (point C); and so on. All combinations of *$Drug X* and *$Drug Y* that lie on PHARMAC's budget line are affordable. PHARMAC's problem is to decide which spending allocation is 'the best'. (This, of course, depends on which of the resulting allocations of QALY gains to *Patient Group X* and *Patient Group Y* respectively that PHARMAC decides is 'the best'.)

Figure A1: PHARMAC's budget constraint



[25] This is closely adapted from Devlin & Hansen (2000), and the diagrams are virtually identical to ones in Culyer & Wagstaff (1993) and Culyer (1995).

[26] The principles developed below extend easily to higher numbers of pharmaceuticals, but they cannot be illustrated graphically (i.e. two dimensionally).

2. The effectiveness of the pharmaceuticals — the 'health production function'

Each dollar spent by PHARMAC on *Drug X* or *Drug Y* produces an increase in the health of one or more of the individuals in the particular patient group that receives it — i.e. an increase in *Patient Group X's QALYs* or *Patient Group Y's QALYs*. Realistically, however, the magnitude of these QALY gains diminish (but still remain positive) with each extra dollar spent, because of the inevitable decline in the marginal effectiveness of the pharmaceuticals (explained more fully below). The possibility of 'over-treatment' in the sense that the marginal effect on health becomes negative (e.g. due to iatrogenic illness) could easily be demonstrated in this context too.

In addition, pharmaceuticals are but one of the many determinants of an individual's health (in QALY terms). Other determinants include other pharmaceuticals (i.e. other than *Drugs X* and *Y*) and other types of health care, diet, exercise, genetics and a host of environmental and other lifestyle factors. In general, therefore, individuals who do not consume *Drug X* or *Drug Y* will nonetheless have a stock of QALYs (i.e. a life expectancy of a certain health-related quality).

These features, in the context of the two patient groups, *Patient Group X* and *Patient Group Y*, are represented by the 'health (QALY) production functions' in Figures A2 and A3 below. The respective vertical axes intercepts, h_x^0 and h_Y^0, reflect the assumption discussed above that without *Drug X*, *Patient Group X* still has h_x^0 QALYs and without *Drug Y*, *Patient Group Y* has h_y^0 QALYs. The positive but decreasing slopes of the QALY production functions (see Figures A2 and A3) represents the above-mentioned positive but diminishing marginal effects on *aggregate* health status of increasing amounts of pharmaceutical spending. At the aggregate level, this can be justified in two ways.

One, each increment in pharmaceutical spending is allocated evenly amongst the individuals in a particular patient group, each of whom, as mentioned above, exhibits diminishing marginal (health care) productivity in terms of health (QALYs). Or, two, individuals in a given patient group with the greatest capacity to benefit from the health care are treated before individuals with lesser capacities to benefit.

Either way, as well as having different intercepts (h_x^0 and h_Y^0) , the health production functions are assumed to have different curvatures. Specifically, for small amounts of spending, the QALY production function for *Patient Group X* is steeper than the QALY production function for *Patient Group Y*. This indicates that *$Drug X* (spending on *Drug X*) is initially more effective at raising health at the margin than is *$Drug Y*, reflecting a difference in the relative capacities of the individuals in the groups to 'benefit' from pharmaceutical spending.

Thus, overall, Figures A2 and A3 indicate that in contrast to their relative health needs (where *Y* is 'needier' than *X*, as noted above), *Patient Group X* has a greater capacity to benefit from treatment than *Patient Group Y*. As noted in the main text, this contrast between the patient groups' 'needs' and 'capacities to benefit' is deliberate, as it serves to most starkly illuminate the differences between common value judgements adopted by decision makers.

Figure A2: QALY production function for $Drug X

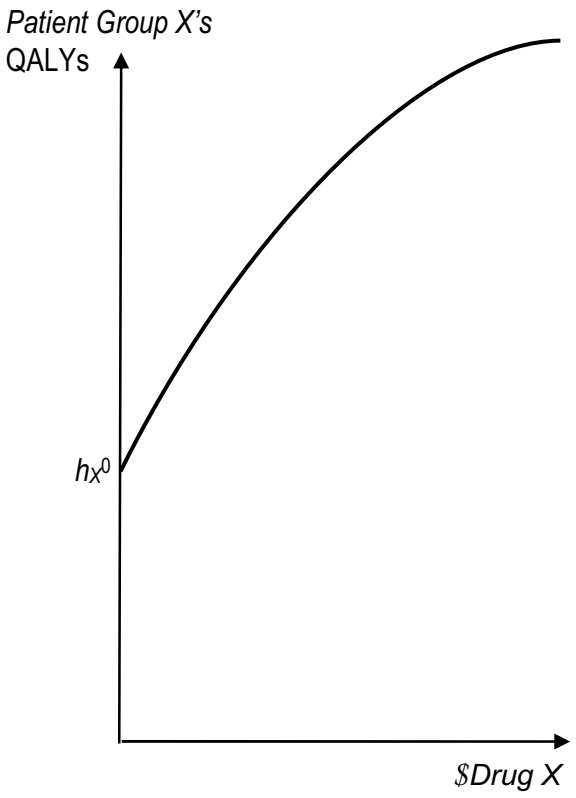
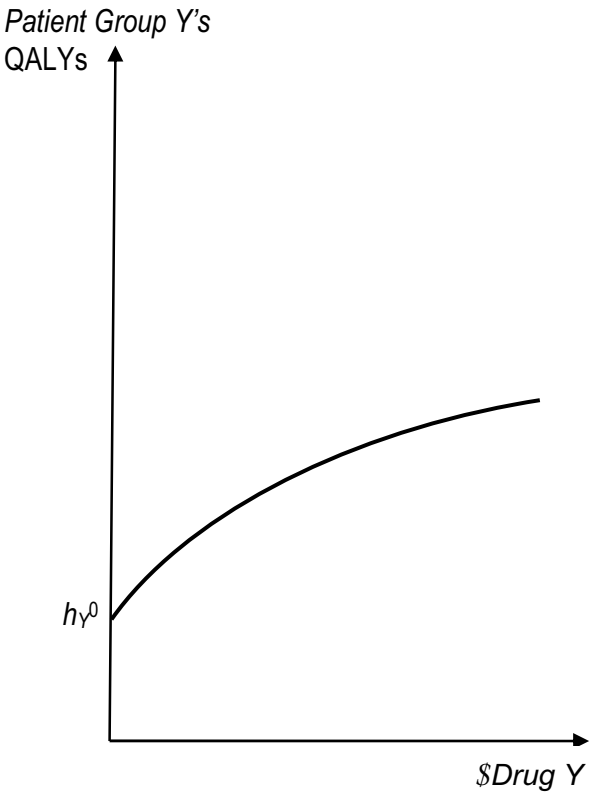


Figure A3: QALY production function for $Drug Y



3. The maximum combinations of health possible from PHARMAC's budget? — the 'health possibilities (efficiency) frontier'

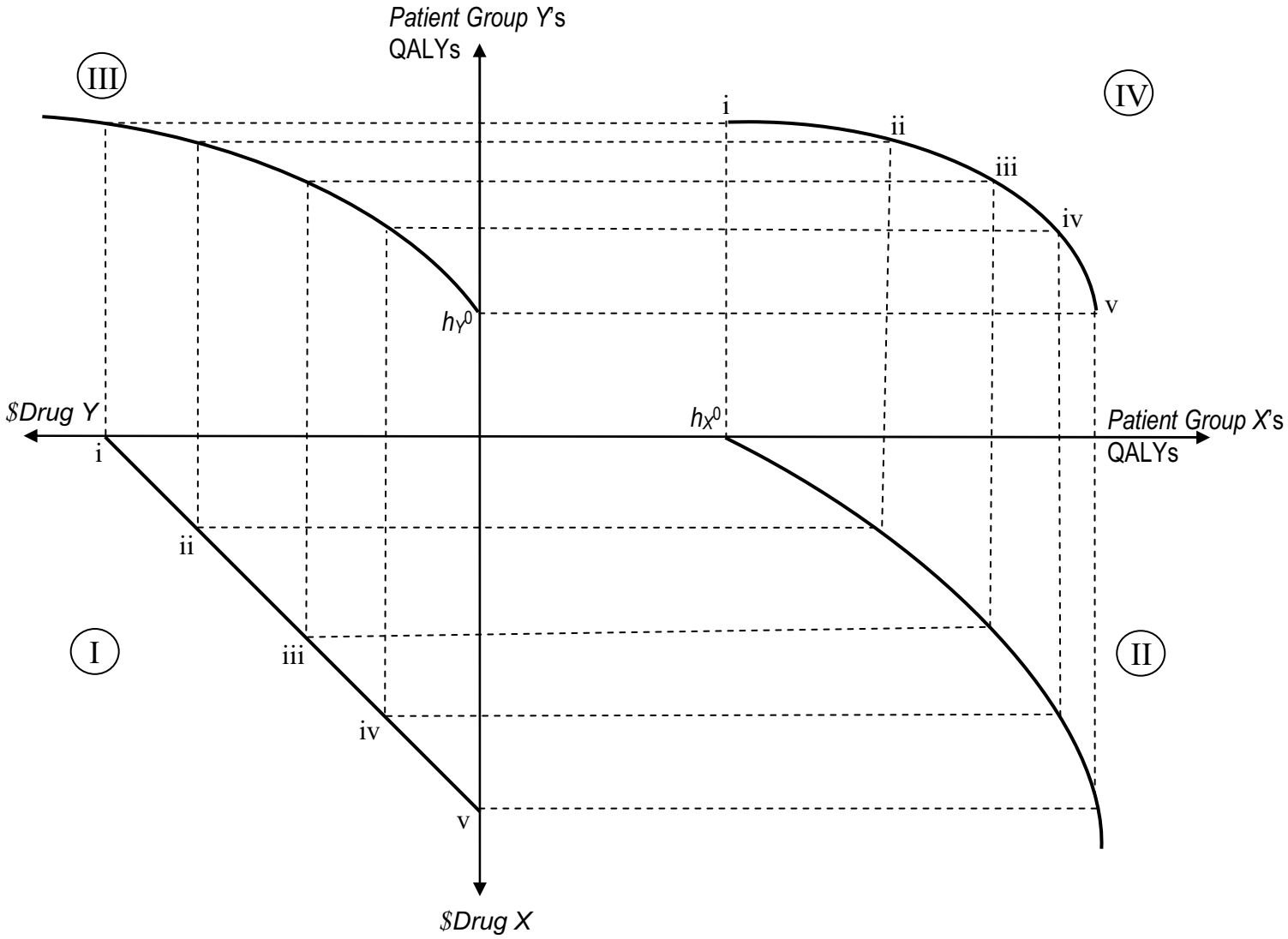
Figure A4 combines Figures A1, A2 and A3 to trace out *all* combinations of health (in QALY terms) for the two patient groups that are affordable with PHARMAC's budget. As mentioned in the main text, this curve is often referred to as the 'health possibilities' or 'health efficiency' frontier. This is the same frontier as in Figures 1 and 2.

Thus, starting in quadrant I of Figure A4, all feasible divisions of PHARMAC's budget between *$Drug X* and *$Drug Y* are shown (the budget line from Figure A1). If you were to choose a particular allocation between *$Drug X* and *$Drug Y* on the budget line in quadrant I (five are shown, labelled i to v), and 'follow it' via the dotted lines as it is transformed via the health production functions in quadrants II (from Figure A2) and III (Figure A2), you would identify a point on the health possibilities (efficiency) frontier in quadrant IV. If you did this for all points on the budget line in quadrant I, you would trace out (via quadrants II and III) all points on the health possibilities function in quadrant IV.

Thus the 'menu' of all affordable QALY combinations for the two patient groups are revealed — from which, as discussed in Section 1 of the main text, PHARMAC must choose one.

Note that the shape and position of the health possibilities frontier that is derived depends on the particular health production functions assumed — distinguished by 'need' (intercept) and 'capacity to benefit' (slope), as discussed above — as well as the PHARMAC's budget and the prices of the two pharmaceuticals p_X and p_Y. Any change in these variables results in a new frontier.

Figure A4: PHARMAC's budget constraint (quadrant I), X's QALY production function (II), Y's QALY production function (III), and the health possibilities frontier (IV)



Appendix B: Overview of the Ministry of Health's Use of 1000Minds Software/Method

Historically, in New Zealand and most countries internationally, demand for elective health services has typically exceeded their immediate availability. Prioritising patients, usually via waiting lists (or 'booking systems'), is therefore inevitable.

Prior to their overhaul in 1998, New Zealand's waiting lists were "a diverse mix of patient cases – placed and kept on the list for a number of different reasons, and with no agreed criteria for admission to the list." (National Health Committee 1996). Patient access was also often inconsistent across regions and specialties.

In 1998, Clinical Priority Assessment Criteria (CPAC), often implemented as points systems, were introduced to remedy these problems. However, the validity of the patient rankings (and the resulting health outcomes) produced by some of these original points systems has been seriously criticised (e.g. Jackson et al. 1999, Seddon et al. 1999).

Since 2004, using Internet-based software known as 1000Minds (formerly *Point Wizard*), the Ministry of Health led several projects to create and validate new points systems (and where possible revise existing CPAC tools), with the goal of more equitable access to elective services and improved patient outcomes overall.

In collaboration with the New Zealand Region of the Cardiac Society of Australia & New Zealand (CSANZ), this was done first for coronary artery bypass graft (CABG) surgery. A group of CSANZ cardiologists and cardiac surgeons in different locations throughout New Zealand used the 1000Minds software via the Internet and a teleconference to create two points systems for determining access to CABG. These points systems has been formally accepted by CSANZ, and CSANZ intends also using 1000Minds to prioritise patients for heart valve surgery.

The validity of the new points system was evaluated by the participating clinicians examining the face validity of the relative importance of the criteria implied by the point values, and also by comparing the overall ranking of sets of patient case descriptions ('vignettes') produced by the points system with the rankings from the clinicians' unaided expert judgements (effectively, the 'gold standard' here). The points systems passed both tests. In addition, a survey of the clinicians involved in scoring the CABG points system revealed high levels of satisfaction with the 1000Minds scoring method/software (The New Zealand Region of the CSANZ 2005).

Other professional bodies, also supported by the Ministry of Health, have also used 1000Minds for prioritising patients for hip and knee replacements, vascular surgery, gynaecological and infertility treatments and cataract surgery respectively, with similar results.

Based on this overall body of work, 1000Minds/*Point Wizard* won the *Telecommunications Users' Association of NZ Healthcare Innovation Award 2005*, and was a finalist for two other (independent) awards: *Global Entrepolis@Singapore Award 2005* (in association *The Asian Wall Street Journal's Innovation Award*) (Wagstaff 2005) and the *2006 NZ Health Innovation Awards*.

Working together as Development Partners, the Ministry's and 1000Minds' goal of further developing a fully-integrated prioritisation process supported by information technology has been realised with such a process currently being implemented.

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Review of the Reports to PHARMAC by Paul Hansen and Raanan Gillon

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Introduction

PHARMAC has requested a critique of one or both of the reports by Paul Hansen and Raanan Gillon concerning the funding of high cost pharmaceuticals. Rather than attempt to revisit each of PHARMAC's questions in turn, the following comments are simply responses to some of the points made in the two reports. Most of my comments relate to the economic aspects of this question - and hence primarily to the report by Paul Hansen. However I also refer in places to aspects of Raanan Gillon's report. I start with some general impression and comments. I then go on to discuss – in no particular order – some issues raised in the reports.

Some general comments

Overall I think both reports are valid and useful, and are firmly grounded in the theoretical and philosophical frameworks associated with the discipline of each of the authors. Neither author attempts to provide any clear cut answers to most of the questions posed by PHARMAC. This seems appropriate, given that most of the questions just don't have clear cut answers! Even so, both Hansen and Gillon propose a process whereby PHARMAC might itself begin to answer some of the questions that they themselves pose.

A key issue which inevitably exacerbates any decisions that PHARMAC must make about HCPs is of course that pharmaceuticals are only one input into health care. Even where a health problem can be addressed solely by taking a drug, a person still has to get access to that drug. If, after taking distributional issues into account (for example via Hansen's four-step process) PHARMAC decides to fund an HCP because it considers that *potentially* it will benefit those who are worst off, the *actual* outcome may be quite different if those most in need cannot (for whatever reason) get access to the health provider that prescribes that drug. Similarly (as I am sure that PHARMAC is acutely aware), the *actual* impact that funding an HCP will have on health

outcomes depends as much on the decisions of prescribers as it does on the decisions of PHARMAC.

These are issues which stem from the funding and structure of the health system more widely. Over the past 10 years or so, numerous people and organisations within the health sector have been involved in efforts to make prioritisation processes more systematic and transparent.[1] These include the Health Funding Authority, the Ministry of Health, most of the DHBs and some of the PHOs as well as PHARMAC. Most of these efforts have incorporated reference to some set of underlying principles. Inconsistency between different principle-based prioritisation processes must inevitably lead to major losses in efficiency and justice. PHARMAC might therefore be wise to consider also what impact the principles and processes of prioritisation in other parts of the health sector might have on access to HCPs. Even the most sophisticated method of making decisions about HCPs may be all for nothing if those decisions are effectively undermined by other aspects of the health system.

Hansen's analytical framework

The analytical framework utilised by Hansen is usually referred to by economists as the "production possibilities frontier" but for the purposes of this particular question has been called "the health possibilities frontier", or "health efficiency frontier". The framework is most appropriate for the purposes of understanding decisions about high cost pharmaceuticals in a budget-constrained environment. It illustrates well how the application of different theories of justice – and hence different expenditure decisions - affect the level of health that is attainable by allocating funding between drugs which have different costs-per-QALY. Incorporation of the budget constraint into the framework also illustrates very clearly how decisions which include some

[1] Reports on these include: Hadorn, D. and A. Holmes, *The New Zealand priority criteria project. Part I: Overview.* British Medical Journal, 1997. 314: p. 131-134. Ashton, T., J. Cumming, and N. Devlin, *Prioritising health and disability services: principles, processes and problems. A report to the National Health Committee on the HFA's proposed prioritisation process*. 1999, National Health Committee: Wellington. Neutze, J. and D. Haydock, *Prioritisation and cardiac events while waiting for coronary bypass surgery in New Zealand.* New Zealand Medical Journal, 2000. Health Funding Authority, *Overview of the health funding authority's prioritisation decision making framework*. 2000, HFA: Wellington.

consideration of the *distribution* of QALYs might differ from consideration of decisions made on QALY maximisation alone.

In the example used by Hansen (in his Fig 2), the different cost-per-QALY occurs because there are differences in the capacity of the two patient groups to benefit, rather than in differences in the price of the two drugs. This is a useful scenario as it highlights the fundamental question: Should those with poorer health status be treated more favourably?

The framework also highlights the fact that the size of the opportunity cost associated with funding HCPs will vary, not only according to the cost per QALY gained of different drugs but also according to the value judgement that PHARMAC uses to guide its decision-making. In some cases, a very small gain by the patient group that benefits from the HCP may imply a very large potential loss of QALYs gained by other patient groups because their drug cannot also be funded. The four-step process suggested by Hansen is helpful in this regard, because it should makes these trade-offs between additional QALYs gained and other potential benefits somewhat more transparent.

A QALY is a QALY is a QALY

On p4, Gillon suggests that "….they [i.e. QALYs] afford no basis for distinguishing between QALYs gained in the context of meeting need, and QALYs gained in the context of absence of need". This comment is, in my view, something of a red herring for two reasons. First, most of the scales (such as the EuroQol) that underpin the QALY algorithm focus on dimensions of physical and mental health. In the example given, while one's mental well being may indeed be enhanced by a visit to the South of France this is unlikely to be reflected in any change in one's utility value as measured by most of these scales and so will not result any gain in QALYs. Second, it is certainly true that a QALY gained is the same, regardless of the cause. However in the particular example given (of an insomniac and heart failure), if a drug for heart failure is life saving, then the QALYs gained from this drug would normally be significantly higher than any QALYs gained from improved sleeping patterns.

Of more relevance for the questions posed by PHARMAC is the fact that QALYs do not distinguish between the people or group to whom the gains accrue. For example, in the QALY algorithm, a gain in utility for a person in a poor health state (i.e an improvement in health status) from a utility value of 0.2 to 0.4 is equivalent to a gain in utility for a healthier person from 0.8 to 1.0. The issue of distribution of health gains between groups with different levels of health status is addressed in detail by the comments from both authors regarding alternative theories of justice. However I return to this issue briefly in my discussion of the four-step process.

Process versus outcomes

A key determinant of the number of QALYs gained from any intervention is the stage of any disease path at which the underlying health states are measured. In most cases, analysts tend to base their estimates of QALYs gained on the difference in reported utility values before and after any intervention, with "after" being the time at which the maximum expected gain would normally be achieved. If this is the case, the experience of the patient during the intervening period is not generally reflected in the resultant measure of "QALYs gained". Even well-designed studies which record and report variations in health status during the recovery period (such as side effects, level of pain, etc.), do not generally include these variations in the estimate of QALYs gained. Thus in considering the circumstances under which there might be justification for funding HCPs, PHARMAC might also consider whether any QALYS gained from higher intermediate levels of health status are worth the additional (opportunity) cost.

A four-step process

I am attracted to the four-step process proposed by Hansen. Such a system would certainly make PHARMAC's decision-making more systematic. It may also reduce the work load, depending upon what proportion of new drugs actually fall below the threshold level. A potential criticism is that it *appears* to place too much emphasis on the cost-effectiveness criterion, because this becomes the first hurdle for any new drug to get past. However, the process will, if anything, have the opposite effect because the four-step process effectively give drugs which are NOT good value for money a second bite at the cherry rather than being rejected solely on these grounds.

There are likely to be situations in which the benefits of a drug that is funded because its cost-per-QALY falls below the agreed threshold level accrue primarily to those who would not otherwise be given priority (eg. those in less need, wealthier people, non-Maori, etc.). In these circumstances it will be important for PHARMAC to be able to defend its decision to evaluate a drug purely on the grounds of value for money rather than on any consideration of distribution of its benefits. After all, such drugs will still have a real opportunity cost for other groups, especially if the population group using the drug is large.

Another potential downside of Hansen's four-step process is that it may be vulnerable to some manipulation. I am not sufficiently familiar with PHARMAC's negotiations processes to comment in detail. However it could be that, once it becomes known that any drugs with a cost-per-QALY above some given threshold will still be assessed against PHARMAC's other criteria, the downward pressure on prices may be reduced, at least for those drugs that are likely to score highly against these other criteria.

Weighting the criteria

I agree with Gillon that Hansen is inconsistent in first, rejecting any attempt to explicitly weight a range of ethical and/or social criteria, and then offering the use of a tool that uses a points system to do precisely that! (I also agree that it would certainly be worthwhile trialling the tool as a research study.)

For any drugs within the reviewable range of cost-per-QALY, my personal preference (and this is purely a personal opinion!) would be to follow Gillon's suggestion of undertaking the process of assessment of drugs against any other criteria *implicitly* while making *explicit* the moral values that underpin any decisions. This would reduce any potential manipulation of the system. The particular issues and evidence that were considered by PHARMAC during the assessment process should also be clearly documented for each drug individually and be available to the public on request.

An allocation committee

The single conclusion to be drawn from this exercise - and all other previous exercises on prioritisation of health funding – is of course that there are no easy answers to how

decisions on health care priorities *should* be made. However, such decisions *are* made on a daily basis. I am no fan of layers of bureaucracy. However from the experiences in the UK with the NICE Citizen's Council, there may be some merit in establishing an independent committee to review decisions where, as Gillon puts it "contentiousness is anticipated".

Commentary on papers by Raanan Gillon and Paul Hansen on High-cost pharmaceuticals review

Sandra Coney
5 April 2006

Introduction

This commentary is written by a non-medical person who will approach the topic from a consumer perspective. However, it is my own personal view.

1. Background to resource allocation

Systematic explicit approaches to resource allocation have been attempted before in New Zealand, most notably by the now disbanded Core Services Committee. This involved a number of public consultations including 'town hall meetings' which canvassed the views of the public. The Health Funding Authority also began a process of 'reprioritisation' before it too was disbanded.

In general, these processes have been fiercely contested and no consensus emerged. It is clear that there is no simple answer to the issue of allocation of constrained resources, and no accepted methodology which is able to reflect the scope and complexities of the range of approaches and values that are brought to bear on this issue.

The author of this commentary similarly does not come up with a path out of the maze. Rather, the focus of this brief paper will be on highlighting issues that arise from the reports by Gillon and Hansen, from a consumer perspective.

2. High-cost medicines, all medicines and the wider health sector

I would like to raise the distortion that occurs through looking at high-cost pharmaceuticals in isolation from all pharmaceuticals, and, taking an even broader approach, the distortion that occurs when medicines are subject to rationing differentially from other areas of the health care sector.

2.1 High-cost medicines and other pharmaceuticals

Looking at the wider context of pharmaceutical use, there is a degree of over-use of pharmaceuticals in particular areas. I will give an example of one area with which I am familiar. Through the 1990s, many thousands of mid-life New Zealand women began to use hormone replacement therapy for prevention and 'wellbeing'. This was the result of aggressive marketing by the pharmaceutical industry, as well as promotion by medical opinion leaders and uncritical dispensing by prescribers. A study in 1997 showed that 20 percent of mid-life New Zealand women were using HRT, an increase from 12 percent in 1991. There was also an increase in long-term use for prophylaxis (North and Sharples, 2001). In 2001, after several decades of use, HRT was definitively shown to cause harm. I am not aware that anyone has quantified the cost to the Vote: Health of the over-use of HRT that occurred for the decade or so

before the publication of the Women's Health Initiative results in 2001, but it would be an interesting exercise.

There are other examples, especially in areas where a 'mass treatment' approach has been taken. The mass treatment of hypertension and hypercholesterolaemia in the 1980s are examples. (Jackson and Kawachi, 1992, and Kawachi, 1992).

Over-use can also occur for historic reasons. Pharmac has tried to curb widespread inappropriate use of antibiotics and, historically, there has been an overuse of some types of pharmaceuticals amongst the elderly, in particular, anti-depressants and sleeping tablets.

In summary, there is still room for improvement in the prescribing of drugs other than high-cost drugs. Such an improvement is largely beyond the role of Pharmac, as it involves such things as regulation of the activities of pharmaceutical companies, funding for clinical research, medical education, consumer information, and the current structure of primary health care.

More rational and judicious prescribing behaviour would alleviate pressure on the whole pharmaceutical budget, such that the use of high-cost pharmaceuticals might not be so problematic.

2.2 High-cost medicines and the wider health sector

There is no logical reason why high-cost medicines should be subject to a set of rationing criteria which do not equally apply to other agencies and health care services, such as treatment services, and the activities of the Accident Compensation Corporation.

The 'booking system' is a form of rationing in the treatment sector, but it only applies to particular treatments, whereas others are not rationed, or at least, not explicitly.

Some very costly treatments, such as neonatal intensive care, are 'over-used', having evolved into almost a competition to salvage the lowest weight babies.

There is no logic to spending six figure sums on severely compromised neonates or people injured in traffic crashes, but denying costly pharmaceutical treatment to others, who may well benefit more, simply because they fall within a different funding silo.

If rationing is to be practised, it should occur evenly across the health sector. I am not convinced that it need occur at all, if more attention was given to practising evidence-based medicine. I am not aware of any work that has occurred to evaluate whether an evidence-based approach would result in resources going further, but I'd like to believe that they did.

If prescribing was more constrained and rational (evidence-based), there could well be savings that could be applied to widening access to high-cost pharmaceuticals that do benefit people, who are often facing serious health problems.

3. Should high-cost medicines be available?

I am in general agreement with the proposition that high-cost medicines should be more available. Even the term 'high-cost pharmaceuticals' is contestable. The level at which Pharmac considers a medicine 'high-cost', differs from some others countries. For example, NICE seems to regard high-cost medicines as those with a cost-effectiveness in excess of 20,000 to 30,000 pounds per QALY (NICE 2005). In other words, there seems to be little agreement as to what constitutes 'high-cost', so that treating high-cost medicines differently from other pharmaceuticals is a proposition that is in itself open to challenge and debate.

There is a strong case to be made for making high-cost medicines more available. People needing high-cost medicines often face conditions or diseases that are rare, life-threatening or have a major impact on their lives.

It would appear at first glance that in this case the decision criteria should be weighted towards the degree of benefit to be gained. If the degree of benefit is significant, then that would justify the greater cost. It would even be possible to argue that a great benefit should be required of high-cost medicines.

However, it is not that simple. Indeed, it may be that for at least some high-cost medicines, weaker evidence, or less benefit, might be acceptable given the situation of many of the people seeking high-cost medicines.

Some of these high-cost medicines are, at least initially, supported by less than robust evidence. While I would ordinarily argue in favour of having good quality evidence for safety and effectiveness before scheduling a medicine, I think an argument can be made for relaxing the standards when people are facing dire consequences. When people are in extremis, such as facing death, they may be prepared to accept a greater degree of risk, and I would probably argue that society should support them in these actions. (This should be distinguished from supporting interventions that are futile).

High-cost medicines may inherently have less robust supporting evidence. They are usually developed to treat conditions that are relatively rare, and/or those that have shown themselves to be intractable to other forms of intervention. The situation of people who are seeking such medicines is often urgent. Consequently, there is an argument that they do not have the luxury of waiting for the accumulation of the good quality long-term studies that we would normally expect to support the approval of a subsidy.

I am not coming to any conclusion here, rather I am putting forward arguments as to why one might consider a different approach to high-cost pharmaceuticals.

4. What outcomes should be sought?

I am in broad agreement with the proposition by Gillon 'that equals be treated equally and unequals be treated unequally'. Both Hansen and Gillon cite Rawls' theories which would probably best express my own viewpoint.

Pharmac decision criteria do not explicitly require it to reduce inequalities, but the agency is required to include the Government's priorities for health funding. A principle in the *New Zealand Health Strategy* is 'an improvement in health status of those currently disadvantaged' and to this end the Strategy seeks accessible and appropriate services for people from lower socioeconomic groups, Maori and Pacific people (Minister of Health 2000).

Ministry of Health research shows that there are significant ethnic group differences in access to medicines. In a 12-month period, European/Pakeha adults were more likely to be prescribed 10 or more items than adults in the Maori Pacific and other ethnic categories. This is surprising when considering that Maori and Pacific people have poorer health status than Pakeha, and Maori are more likely than Pakeha to have six or more visits to a GP in a year (Ministry of Health 1999).

Therefore a case might be made for greater access to high-cost pharmaceuticals for these groups who have received less at the primary care level, and whose health status is generally lower. . The argument for distributive justice could support greater accessibility to high-cost pharmaceuticals for those who have missed out on first-line treatment, or who are the victims of structural inequality. Because differentials based on ethnicity are generally unpalatable to governments and to the public, this could, in a circuitous way, bolster the case for a general widening of access to high-cost pharmaceuticals.

Cost Utility Analysis inherently discriminates against those with chronic illness, people with some disabilities, old people and others who will never get 'well'. The quality of life of some of these groups may already be compromised and these same people are often subject to other forms of discrimination and marginalisation in their daily lives. In the case of the elderly, they are means tested before receiving care which does not occur with any other section of the population.

In the case of the elderly, or others, I am not arguing for heroic and futile treatment, but for ready access to pharmaceuticals which will improve their quality of life, even if they do not prolong it or even if they cannot cure underlying conditions.

Indeed, any system of allocating health resources should not place undue emphasis on prolongation of life, at the expense of quality of life in the present. The QALY system inherently does this.

Even the concepts of improving health status or addressing 'need' are problematic for already compromised groups such as those I have discussed. For example, imagine a drug was developed that would assist continence in the elderly. Would a person with dementia, in a wheel chair, have improved health status if treated? Would their 'need' be greater than a person who could get back to work if given a particular drug? Probably not, but there would be improvements in the degree of comfort of the person, there would be less likelihood of rashes and skin irritation, and it would be more feasible to take them on trips outside the institution thus enhancing their quality if life.

The person's autonomy and dignity would be respected, and as a society we would be showing that all human life is valuable, especially our most dependent and vulnerable members of society.

As Gillon argues, the priority of pharmaceutical policy should not always be to save life ahead of other priorities for improving the quality of people's lives.

There will be a range of views about this among the public, but very often the public does not support saving life at all costs. I was struck the other night by a mother interviewed on TV (in the case of her child damaged during birth) who said she had hoped her child would not live, and even though she now loved him greatly, she still wished he had not lived because of the low quality of his life and his very bleak future.

I think we often do not given the public due credit for their understanding of the complexities of these issues, and their acceptance that sometimes saving lives condemns those saved to a life of suffering.

5. The rule of rescue

There are real dilemmas around the urge of society to save people under imminent threat. While, as Gillon says, it is to a degree 'instinctive', it is in our society highly manipulated and can result in actions which exacerbate inequalities.

The rule of rescue is exploited by the media which in recent years loves a 'human interest' story especially one which involves bashing unfeeling bureaucracy. Examination of the evidence for effectiveness comes second to the human drama.

More recently, the internet is being used to build support for particular sad cases. While this can be seen as 'democratic', as it is accessible to many people and it is unmediated by any authority, it can also be a forum for the exchange of uninformed, inaccurate information, and it appeals to other aspects of the human character than reason and logic. This is not necessarily a bad thing, as human being should give weight to values such as compassion, community, charitableness, and kindness to strangers. However, it can build into public campaigns that benefit particular individuals, regardless of the actual merits of the case, and leapfrog over other people with less compelling and dramatic cases, or with fewer resources to draw their situation to public attention.

There are strong moral and social status elements in most appeals to the rule of rescue. The disease is usually seen as particularly tragic and random, and the sufferer has usually done nothing to bring it down on themselves. For example, it would be hard to build a campaign around a still-smoking sufferer from lung cancer.

The sufferer is often educated and middle class with social networks that are able to bring many professional and personal resources to a campaign.

For these reasons, responding to these situations has the potential to exacerbate existing inequalities in health status and access to services.

On the other hand, sometimes, this is all people have left to do, and they are facing life and death issues. A rigid application of rules runs the risk of demeaning society as brutal and uncaring. I do not think anyone gained from the refusal to give dialysis to Rau Williams, which was traumatic and depressing.

In this case, politicians supported the stance of the bureaucracy. On other occasions, they overturn decisions of the bureaucracy. Some see this as a bad thing, and sometimes it is. But it is the role of politicians to step in and intervene when bureaucracies are acting in rigid and inhumane ways. Politicians are accessible to the public, and accountable to them. After all, all our bureaucracies are the creations of politicians, so they do have a duty to listen to the public, assess the situation and intervene on behalf of the public when they think the bureaucracy has got it wrong. The bureaucracies are the servants of the people, not their master.

The way in which society cares for its most vulnerable is an important measure of how humane and mature we are as a society. This is recognised in many aspects of our social, legal and political structures and in our daily interactions with each other as human beings. Adults in general watch out for children, even where they are not known or related. In general, we support treating elderly people with respect and care, so they are comfortable in their final years, although we do not always do this very well. There is general consensus that we provide support and resources for people with disabilities, who are sick or who have suffered misfortunes such that they cannot be totally self-supporting.

Although some of these community values have been eroded in recent years, they are important, and government policies and practices should bolster rather than erode them.

6. So how do we decide about high-cost pharmaceuticals?

Hansen makes the point that Pharmac's existing methodology is focused on outcomes, and that an economics-based methodology is utilised to inform decision. The Cost-Utility Analysis is concrete and well-developed. This is but one aspect of the decision-making, and other aspects can be brought to bear on final decisions by Pharmac.

However, because the other factors that can be considered are vague and not codified, I suspect that Cost-Utility Analysis is fairly determinative. (I concede that I have not seen any research to back up this assertion.)

The process by which decisions are finally taken is not transparent, and they are not (that I am aware of) well documented and therefore open to scrutiny.

Hansen's proposed mathematical approach to making decisions, and the hypothetical points system for deciding which pharmaceuticals to fund (Figure 3, p 23) do not sit comfortably with me. Allocating points on the basis of age is repugnant and I would think illegal within the New Zealand human rights framework.

While a quantitative system is superficially attractive, there is little evidence it will lead to good decisions. It may even act as a refuge, allowing us to avoid taking

responsibility for the decisions that are made. We must never lose sight of the fact that real people with families and friends are affected by such decisions. If we were to have a tick-box system, can I suggest that the final line is:

'Would you want your child/father to have access to this drug if he needed it?'

I would argue that because such decisions critically affect human lives, it is better that they are made through a more deliberative process, that allows other aspects to be considered alongside cost, efficiency and cost-effectiveness.

I am arguing that making such decisions should be values-based, rather than made according to a formulae. While assessment of costs is important, and should be part of the decision-making, it should only be one part and there should be an explicit process for ensuring that other values play a significant part.

It is also important to be more explicit and open about the process. Decisions should preferably be made in public, or at least made public, and the basis of the decisions needs to be well explained.

I am arguing that the process by which these decisions are taken is just as important as the outcome. I would hope that if people understand and respect the process, they would understand and support decisions that are taken.

I am making an argument for a much more people-based system. One where the decision-makers know just who they are affecting, and where the decision makers are known and accountable for their decisions.

Gillon recommends the creation of an 'allocation committee' although he sees it as advisory to Pharmac rather than making decisions. He suggests looking at models such as ethics committees, and the NICE Citizens' Council.

There are merits in the Citizens' Council model. It is an attempt to incorporate the values of the public. Efforts are made to ensure that the council is perceived as having independent standing and is not a tool of the allocation entity. The selection of the councillors is undertaken at arms' length from NICE, meetings are held in public, and councillors help choose witnesses and question them.

I personally have some issues around the make-up of such a council, and members' accountability for the effects of their decisions.

A Citizens' Council is constructed of notional demographically representative citizens, so that the council mirrors the make-up of society by age, ethnicity, gender etc. However, a single person cannot represent the totality of the viewpoint of all those in the same demographic group, so that it is questionable how 'representative' such a committee can be.

I would prefer, rather than the unaligned 'man or woman off the street' approach, that people are nominated by respected community organisations, so that they have access to a broader range of views than their own, and they have experience of a constituency. While fears have been expressed that representatives of community

groups will only advance the agenda of their groups, this is not so in practice. Using people who have a constituency has been shown to result in better decision-making than the 'lay person ' approach, and there is evidence that the public recognises representatives of civil society as representing their interests (Coney 2004).

I am not sure the exact Citizens' Council model would be acceptable in the New Zealand context. The UK puts great store in the NHS on the notion of citizenship. Such a concept is somewhat problematic in New Zealand because of the existence of the Treaty of Waitangi.

Another alternative would be to elect people so that they are directly accountable back to the public they represent.

There is value in having some continuity of individuals on any allocation entity. There is merit in looking at the composition of ethics committees as one form an allocation group could take. Without being categorical about this, I think people with ethical, legal, human rights, and other backgrounds should be included in any allocation process. Consumer representation should also be included.

Some means of accountability for the panels should be developed. This could include a publicly known process for appointments, opportunities for people to express an interest in being included, as well as nomination by various bodies, such as health professional colleges. The general features of decisions should be publicly reported, and the panel could publish an annual report, summarising the year's work.
It would also be important that such a council deliberated in public, to reinforce transparency and accountability.

There could well be few takers for such positions!

Gillon also recommends making explicit the ethical framework in which Pharmac works and I support the development of such a framework. I would see Pharmac as needing to consult widely in the development of a framework.

In doing this, Pharmac could well look at the recent *Social Values Judgements: Principles for the development of NICE guidance* (NICE December 2005). These provide guidance on how to incorporate social values into allocative decision-making. They were developed from a review of published literature, reports of the Citizens' Council and a telephone survey of the public about the role of NICE and attitudes to priority setting. They provide a basic set of 'bottom-lines' for decision-making to safeguard against decision-making being discriminatory or judgemental. For example, Principle 7 states that decision-makers must not distinguish between individuals on the basis or gender or sexual orientation. (New Zealand might come up with a different set of principles than these, so I am not arguing that the NICE principles are simply transferable).

I think the development of a similar framework as a first step would assist Pharmac in approaching the current topic, but it is essential to do this with external stakeholders and the public, not as an internal project.

Gillon also recommends an appeal mechanism and I would support this.

The recent NICE Social Values Framework arrives at characteristics for strategies for setting priorities that I found very attractive, and the section of the report dealing with this is worth copying here.

> '**2.2 Strategies for setting priorities**
>
> There is a groundswell of opinion among bioethicists and political philosophers that, if there is to be confidence in the legitimacy of decisions, the procedures adopted should have all four of the following characteristics:
>
> - Publicity
> - Relevance
> - Revision and appeals
> - Regulation
>
> **2.2.1 Publicity**
> Decisions about limits on the allocation of resources should be made public. This includes not only the decisions themselves, but also the grounds for making them. It does not, however, require that all criteria for decision-making should be established in advance: rather, there should be room for the development of "case-law".
>
> **2.2.2 Relevance**
> "Relevance" means that the grounds for decisions are ones that fair-minded people would agree are relevant to meeting healthcare needs, especially where there are constraints on resources. In particular, 'relevance' focuses on the importance of deliberation about the limits of the common good and acknowledges that such "deliberative democracy" should involve both the decision-makers themselves and those whom the decisions affect.
>
> **2.2.3 Revision and appeals**
> There must be opportunities for challenging decisions and mechanisms for resolving disputes. There should be system in place for revising decisions when new, or additional, evidence becomes available or new arguments are put forward.'
>
> **2.2.4. Regulation**
> There should be either voluntary or public regulation of the process of decision-making to ensure that it has all three of the above characteristics (publicity, relevance and opportunities for revisions and appeals.'
>
> **2.2.5 Accountability for reasonableness in decision-making**
> Ensuring that procedures have all four of these characteristics makes decision-makers "accountable for their reasonableness". Critics claim that majority preferences – however well-informed and fair – will sometimes lead to unjust outcomes, that deliberative democracy in action will "most certainly" conflict with the principles of justice, and that "deep suspicion is warranted about procedural strategies for setting priorities". Such criticisms have some merit: yet no reasonable theoretical or practical alternatives have been proposed to resolve the conflicting theories of distributive justice.' (NICE 2005)

As a step towards a process that has public acceptance, Pharmac could develop a discussion paper with options for strategies and structures for make recommendations about high-costs mechanisms. This could put forward a number of principles and models, drawing from overseas, but shaped for New Zealand's unique circumstances.

A public dialogue would engage the public in this issue as well as raise public knowledge of its complexities. Independent research, such as that commissioned by NICE, could also assist the process. This would be first step towards developing a process for making high-cost allocation decisions that is known and accepted by the public as a fair way of approaching this very difficult subject.

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Commentary on the High Cost Pharmaceuticals Review.

Reviewer: Matiu Dickson
Date:17 March 2006

Introduction:

I have been asked to review the reports of Professor Raanan Gillon[1] and Dr Paul Hansen.[2] Their reports gave responses to questions put by Pharmac[3] with regard to how to deal with the funding of high cost pharmaceuticals. With respect, it is my view that the reports address the funding process adequately from a western liberal or mainstream point of view but do not deal with the local New Zealand situation and the special position of Maori who are the indigenous people.

While I have some knowledge of Maori cultural practice I do not want to be regarded as the spokesperson for all Maori. My knowledge comes from my own tribal upbringing,[4] more particularly that of my maternal grandparents who raised me in a Maori cultural environment at Matakana Island.[5]

I submit that it is important that any considerations as to the delivery of high cost pharmaceuticals requires a Maori component and input because of the following:

1. Maori have a contractual relationship with the Crown by the Treaty of Waitangi 1840 and they are the tangata whenua or people of the land;

2. Maori cultural practice and belief has been ignored in the past yet despite this, it is still highly relevant and vital. It needs to be taken into account as a criteria for funding to achieve a long term beneficial effect for Maori;

3. Maori are most likely to be the in the group requiring such medications because of their susceptibility to illness at a multiple level;

4. Maori comment and consultation on a wide basis is necessary for decisions likely to affect their wellbeing.

These considerations are not in a particular order of priority nor perhaps are they only considerations, but for now they suit the purposes of this review.

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[1] Emeritus Professor of Medical Ethics, Imperial College, London, England.
[2] Senior Lecturer, Department of Economics, university of Otago, Dunedin, New Zealand.
[3] Pharmaceutical Management Agency, New Zealand.
[4] Ko Mauao te Maunga, Ko Tauranga te Moana, Ko Ngaiterangi te iwi.(Mauao is the ancestral mountain, Tauranga is the life giving sea and Ngaiterangi are the people.) A whakatauaki or proverb identifying the writer's tribal connections to the land.
[5] An island in the Tauranga Harbour whose community is almost wholely Maori.

1. The Relationship of Maori and the Crown.

From a legal point of view the decision in *The New Zealand Maori Council v Attorney-General* [6] broadly established the relationship of Maori and the Crown with regard to the Treaty of Waitangi 1840. Since that important case, this relationship had developed and been redefined mainly for political reasons. It has now been described more succinctly as being that relationship incorporating the principles of partnership, protection and participation.

This interpretation is referred to as being a commitment of Pharmac in dealing specifically with planning documents, and operating policies and procedures, in the Maori Responsiveness Strategy 2002 (the 'Strategy')[7].

In my view this commitment alone means that Maori comment and consultation is absolutely required by Pharmac before a process of funding high cost pharmaceuticals is formulated. Failure to do this makes a mockery of the commitment. Given that the Strategy was intended to improve connections between Pharmac and the Maori community, ignoring this commitment would mean a loss of trust by Maori.

The position taken by Pharmac is further endorsed by its adoption of the Maori Health Strategy (Te Korowai Oranga) released by Government in 2001. Pharmac has therefore undertaken to work with Maori communities to achieve their health objectives.

From a Maori cultural point of view, trust ('pono') is essential to a binding and meaningful relationship. Maori relationships ('whanaungatanga') usually depend on blood ties ('whakapapa') but where this is not possible (very rarely, given the extent of genealogical knowledge) the idea of reciprocity ('utu') applies. That is, that a good and therefore honest deed deserves another in return. Reciprocity in its wider sense formed the basis of interaction between Maori social groups, and in a negative way was the reason for many tribal conflicts before and since the Europeans arrived. The positive aspect of this cultural aspect is often overlooked but survival meant that doing good deeds were the most important of all.

The Crown's relationship with Maori has been very much at the whim and political will of the stronger partner, the Crown. Pharmac has an opportunity to progress from this and has a legal if not moral obligation to do so.

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[6] [1987] 1 NZLR 641. Cooke P in an unanimous decision of the Court of Appeal sought to clarify the relationship between Maori and the Crown according to the spirit and principles of the Treaty. He referred *inter alia* to the principle of partnership and to fiduciary duty requiring both parties to act in the 'utmost good faith and trust towards each other'.

[7] Refer to *Implementing Pharma's Maori Responsiveness Strategy 2006* in which the commitment by Pharmac to the principles of the Treaty are reinforced at p8

As tangata whenua ('people of the land') or indigenous people of New Zealand, Maori perhaps have a moral right also to depend on the Crown and therefore Pharmac to "level the playing field" so that their health statistics compare more favourably to other New Zealanders. Most indigenous peoples throughout the world have had the same colonizing experience as Maori but progress toward a better future for these people has often been abysmal.

Maori Cultural Practice.

The Crown has historically ignored Maori cultural practice and has in some instances actively opposed the teaching and retention of Maori cultural values ('uara'), custom ('tikanga') and language ('reo')[8]. The education system is a case in point. More recently, attitudes have changed for the better but there is still a lingering idea that Maori values do not fit with mainstream New Zealand ideology. And if it were to, it is expected to be a validated by the standards of "mainstream" by mainstream experts. What is needed then is a better understanding and acceptance that Maori values still hold a place and validity even in deciding how to fund high cost pharmaceuticals.

Tikanga is the Maori word for custom, it literally means "the right way" or doing what is right ('tika') for all of the parties, for the time being and *for the future*. Maori ideas of doing what is right are determined by open discussion, inclusiveness and the desire to achieve oneness ('whakakotahitanga') of thought and purpose. The collective good is the main objective.[9]

When compared to the utilitarian ideas in the reports there is a quandary. The alternative Rawlsian theory of favouring patient groups with relatively poor health may be more pertinent for Maori .

Culture therefore ought to be a factor for allocation. The connection that Maori have to their group means that any effort to improve the health of some of its members has a positive effect on the group as a whole. There is an overall utility for the Maori community when its un-well members recover.

Maori Need.

Statistics tell an alarming story of Maori un-wellness and the future does not look to great either. Typically, Maori suffer from illness which is mostly related to lifestyle. Some commentators say that these are the long term effects of colonization.

Progress in stopping this trend almost always points to an effort worked out and applied by Maori themselves. In my view, this is the best alternative before us thus far.[10]

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[8] See Education Ordinance 1847 State funding for schools was given provided the language of instruction was English. The Government followed a policy of assimilation but Maori complained because the choices of vocation for them were only as domestics and farm labourers for the colonists.
[9] See Metge J Korero Tahi-Talking Together, p39
[10] See Dickson M in State of the Maori Nation, Ed.M Mulholland. p187

Such an approach is called ‘kaupapa Maori’ or the Maori way/purpose. The only problem is that those who have the power (the decision makers) and the resources (funding for example) have to make bold decisions to allow Maori the opportunity to try things their way.

Would Pharmac consider a Tikanga Panel of elders (‘kaumatua’) that uses Maori tikanga for making decisions (eg funding processes) affecting Maori health?

Maori cultural practice reveres the elderly or the wise ones. Within Maori society the elders are given positions of respect because they have the knowledge of the tribe. They are thought appropriate to make the decisions that will guide the tribe and maintain its traditions.

The nearest alternative would be for the mainstream decision makers to have some understanding and empathy with the Maori predicament. However, experience shows that this half hearted approach recognizes the problem but does not solve it. More innovative ideas are required.

Maori Input.

As mentioned, Maori input into finding a way to fund high cost pharmaceuticals is essential and must be meaningful and not tokenism.

Ideally, Maori tikanga and its application can assist in a collaborative effort to improve Maori wellbeing and therefore the wellbeing of all New Zealanders since the extra resources for Maori should not be necessary.

It may be thought undemocratic to treat Maori as a special case, but there are very good reasons shown above for considering this course of action. Democracy is also about fairness and equity.

Conclusion.

Throughout this paper I have focused on advocating for Maori and their unique position as I see it, in decisions Pharmac has to make. I make no apologies for doing this because it is important to advocate for Maori and in the long term it is a benefit for all New Zealanders.

It bears repeating that though the reports refer to the western liberal or mainstream practices of sharing and ethics, they do not refer to the special circumstances of Maori and the New Zealand context.

It is highly desirable that Pharmac refer to the undertakings they have already made to the Maori community by the Strategy and, with reference to Maori communitarian norms.

Kei hea te komako e ki nei?
He aha te mea nui o te Ao?
Maku ka ki atu, He tangata, He tangata

(Where is the bellbird that sings?
What is the most important treasure on earth?
I say to you…It is people, it is indeed people.)[11]

[11] A well known proverb that uses the oral tradition to reinforce the unity of man.

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An Ethical Framework for Pharmac's Subsidy Decisions on High-Cost Pharmaceuticals

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1 A Continuing Challenge

In this report I describe how Pharmac might develop an ethical framework for deciding whether and to what extent to subsidise high cost pharmaceuticals in New Zealand.

Although development and marketing of ever-more expensive drugs against cancer and other serious illnesses may represent an advance in patient care, the problem of affordability will continue to challenge patients and payers like Pharmac for many years to come. New drugs developed for serious diseases are almost always priced extremely high, based not on production considerations but rather on a corporate estimate of what the 'market will bear' or on the perceived clinical value of the treatment. Today's New York Times carries a story on this topic, including the following excerpts (see Appendix A for full story):

> After years of defending high prices as necessary to cover the cost of research or production, industry executives increasingly point to the intrinsic value of their medicines as justification for prices. Last year, in his book "A Call to Action," Henry A. McKinnell, the chairman of Pfizer, the world's largest drug company, wrote that drug prices were not driven by research spending or production costs. "A number of factors go into the mix" of pricing, he wrote. "Those factors consider cost of business, competition, patent status, anticipated volume, and, most important, our estimation of the income generated by sales of the product." In some drug categories, such as cholesterol-lowering treatments, many drugs compete, keeping prices relatively low. But when a medicine does not have a good substitute, its maker can charge almost any price. . . . The result has been soaring prices for some drug classes, notably cancer treatments. In 1992, Bristol-Myers Squibb faced protests for its plans to charge $4,000 a year for Taxol, a breast cancer treatment. Now, most new cancer treatments are priced at $25,000 to $50,000 annually. In some cases, companies are pushing through substantial price increases on already-expensive drugs. . . .[i]

(See Appendix B for another recent *New York Times* story on current high-cost drug development, including a *vaccine* for cervical cancer.)

Without adequate restraints in place, public spending on drugs could potentially bankrupt the health care system within the next 10-20 years. Well before this occurs, the ever increasing number of ever more expensive pharmaceuticals will pose a real threat to distributive justice in terms of unfair differences in availability of expensive, effective drugs.

I believe that Pharmac is in a good position to lead the world in developing a policy to deal with the threat to affordability and equity just described. The approach I will outline in this report blends cost-utility analysis (CUA) with the principles underlying New Zealand's priority criteria approach to elective services, i.e., transparency and fairness.

2 Stick to Your Knitting

Pharmac's principal role is to manage the pharmaceutical budget allocated by Government so as to maximise the benefits obtained from drugs purchased within that budget. This is a basically utilitarian approach, which is appropriate for an agency like Pharmac. A "Rawlsian wrinkle" can and should be built in (i.e., a concern for the least well-off) by electing not to take into account the adverse effects of coexisting conditions on health-related quality of life (HRQOL). Such conditions, common in older and lower

income people, tend to reduce the net benefits of treatments because pain and disability are still experienced due to the other health problems.[ii]

This is the sole concession that Pharmac should make to non-utilitarian considerations in the methods it uses for CUA, however. In all other respects, the process should be a straightforward one in which quality adjusted life years (QALYs) are calculated to provide a common currency with which to compare the outcomes of different treatments. All QALYs should be considered equal unless and until someone puts forth a convincing case that some QALYs should be considered more valuable than others, e.g., in view of the perceived urgency of the situation. I do not believe that such a case has yet been made.

Pharmac must not let itself get bogged down in endless debates about such things as whether premiums should be added to some people's QALYs, whether young people's QALYs should be worth more or less than old people's, whether we should strive to accommodate the Rule of Rescue (see below) within the CUA, and so forth. These essentially unanswerable questions represent angels dancing on heads of pins, and they should not be permitted to disrupt Pharmac's legitimate search for a formal protocol for evaluating high-cost pharmaceuticals.

Pharmac should, therefore, I believe, stick to its knitting and continue to perform (or to seek to perform) the most transparent, consistent, and rational CUAs of any health agency in the world. But Pharmac should not try to be all things to all people, and it should resist the urge to 'tinker' with the principles underlying CUA. Pharmac's fundamental obligation is to ensure *efficiency* in obtaining value for public money, and 'value' in this setting must refer to the commonly agreed 'final outcomes' of health care (including pharmaceutical treatment), i.e., (1) length of life (how much longer with treatment?) and (2) quality of life (how much better with treatment?). These outcomes are appropriately captured by the QALY metric.

In its CUA analyses Pharmac should rule 'out of bounds' any appeals to the imminence of death ('only hope' treatments), the visibility of predicament ('Rule of Rescue') or other non-rational (i.e., emotional) considerations (except for the Rawlsian wrinkle discussed above), as these contravene the basic assumption that all QALYs are of equal value.

3 Rule of Rescue

Perhaps the most significance obstacle to a rational approach to subsidy decisions for high-cost pharmaceuticals is the social and political difficulty entailed in saying "sorry, no" to patients (many of whom are desperately ill) and their doctors and advocates whose conditions might benefit from drugs deemed insufficiently cost-effective. This is especially a challenge when patients' conditions are terminal, e.g., advanced cancer.

It is part of human nature to wish to rescue endanger life. Our strong proclivity to save people whose lives are visibly threatened was originally dubbed the Rule of Rescue (RR) by Al Jonsen, who recognized the difficulties posed by RR for health car planners who are attempting to allocate resources:

> Many of the technologies under assessment relieve illness or pain or disability, but do not directly save life, do not rescue people from imminent death. Those technologies that do stave off death pose a particularly daunting problem [which represents] a barrier difficult to climb, a chasm difficult to leap: namely, the imperative to rescue endangered life. . . Our moral response to the imminence of death demand that we rescue the

> doomed. We throw a rope to the drowning, rush into burning buildings to snatch the entrapped, dispatch teams to search for the snowbound.[iiiiv]

The phenomenon is familiar. We observe with approval the expensive rescue of a trapped miner -- even though we would have earlier rejected a utility rate increase for safety improvements that could predictably have prevented the mine accident from happening in the first place -- and at a fraction of the rescue costs.

RR was paradigmatically illustrated when the world's attention was riveted on an infant, Jessica McClure, who spent 58 hours trapped in a well near Midland, Texas. In the four days following her rescue, Midland Memorial Hospital received over 2,000 phone calls (including one from President Reagan) asking about Jessica's condition, and Vice President Bush paid a personal visit. The Associated Press rated the Jessica McClure story the tenth most important of 1987 -- just after the AIDS epidemic and jetliner crashes in Detroit and Denver. A television movie was made of the event in 1989 ('Everybody's Baby: the Rescue of Jessical MacClure) and news agencies continue to report events in her life - including her recent marriage (http://www.cbsnews.com/stories/2006/01/30/national/main1252976.shtml?CMP=OTC-RSSFeed&source=RSS&attr=U.S._1252976).

My paper on the Oregon experience (JAMA 1991)[v] brought RR into wider circulation, while describing how RR effectively prevented the Oregon Health Services Commission from employing a CUA-based prioritisation approach. The process proposed herein for Pharmac takes this experience into account, as described below.

Our perceived duty to identified victims of life-threatening circumstances is far greater than what we feel is owed to mere "statistical victims" -- whose deaths will, however, be no less real. Philosophers who have examined this psychological phenomenon have failed to discern any relevant moral differences between identified and "statistical" victims. Leon Trachtman, for example, has described

> . . . the contrast between designating a single known individual for death and imposing death as a statistical certainty on an unknown and unknowable number of a social group at risk. This resembles the difference between the anonymity of the victims of the artillery man or the bomber pilot and the specificity of the foot soldier's victim -- a visible, tangible body that the soldier must pierce with his bayonet.[vi]

Our tendency to discount the interests of statistical victims "complements" RR as applied to identified victims -- together they result in clearly irrational policies, at least from the standpoint of lives saved and suffering prevented. In the health care arena, for example, we neglect to expend significant resources in disease prevention that could reduce far more premature death and suffering than do belated efforts to cure the diseases or afflictions which predictably occur to ultimately identifiable people in the absence of effective prevention efforts.

The emotion-based RR seems clearly to be at odds with fundamental principles of distributive justice, including the insurance principle, which states that people do not owe each other costly assistance beyond what it would be rational for them to buy insurance against in prospect. That is, people planning a society of which they would be a random member would elect to apportion only a limited amount of resources for insurance against catastrophic problems, preferring to have adequate resources to devote to other wants and needs:

> Most treatments are either worth their cost for everyone or worth their cost for no-one -- whether something is worth its cost is reckoned from the

> prospective, prudential standpoint of whether it would be rational for a person to buy insurance to cover it.[vii]

The problem is, people are not willing to gracefully accept an uninsured loss once it occurs -- we cannot follow through on our gamble that the mine will not cave in or that disease will not occur. We feel compelled, and society with us, to make up for what is now viewed as a bad bargain. Consider, for example, what public reaction would be to a proposed government policy banning the rescue of trapped miners (or little girls) if rescue costs exceeded a certain amount. Such a policy would, of course, be roundly rejected, -- with the primary argument against it being the claim that "human life is priceless" – a praiseworthy but thoroughly impractical sentiment. As noted by American philosopher Alan Gibbard in the President's Commission report:

> If an illusion that we regard life as priceless strengthens the bonds of social fellowship, then whether we should indulge that illusion may depend on how much it costs to do so. With the development of new, effective extraordinarily expensive treatments, we may be increasing the economic cost of maintaining that illusion, and the cost of the illusion may begin to outweigh its benefits.[viii]

Since society can afford to rescue trapped miners and little girls, we should continue to do so. The cost is low and the return high in the form of societal solidarity, reaffirmation of the value of life, and in the comfort we all derive from knowing that similar help would be given to us should we ever need it.

In the area of health care, however, the situation is entirely different. Here "needs" are not occasional, but constant -- nearly everyone can live longer if provided access to life-prolonging interventions. Al Jonsen wonders if we should, therefore, "force ourselves to expunge the rule of rescue from our collective moral conscience"[ix] in making health policy decisions. Allan Gibbard thinks we should at least try:

> Crudely put, what I am suggesting is this: that whereas cheap violations of narrow economic rationality may well be wroth in sentiment what they cost, as violations become costly, we should refine the sentiments involved. To do so is a natural, if painful, result of economic change, and it can often be desirable. One set of changes in our moral sentiments that may be called for by current technology is a refinement of our ways of thinking about risk, and about what we owe each other in the way of extra-ordinarily expensive treatments.[x]

Thus, it might be possible to "refine" RR in light of economic considerations. I do not believe, however, that it is possible for the rule to be "expunged from our collective moral conscience," since the moral and psychological underpinnings of RR are an aspect of the fundamental human fear of death. We are fascinated by the imminence of the victim's death and identify with him or her and with the fear of death that person feels. We therefore feel compelled to symbolically (and actually) "give the gift of life" to him or her if it is within our collective power to do so.

We must, however, recognize that our "prudential tendencies" to rescue the doomed are often irrational and must not be used as a basis for health policy.

In summary, the emotional upheavals characteristic of RR-relevant situations will inevitably contribute to the anguish generally associated with illness and death, especially when patients are 'permitted' to die earlier than is technically possible for want of resources (e.g. drugs) that are available to others but not to them. But it is to be hoped

that the proposed process would distribute anguish more equitably, and perhaps make it easier to bear for its fairness.

What is needed, and what I believe the proposed process may be able to provide (if anything can), is a method by which we can not expunge but rather refine our proclivities to rescue all endangered life so as to realize the greater ideal of a just society.

3.1 Need for Wiggle Room – A Tithing Approach?

One possible concession to the power of RR (and related emotion-based obstacles to the efficient distribution of health care) could come in the form of a 'tithe', under which Pharmac earmarked (say) 5% of its budget for drugs that it views as not cost-effective under its CUA protocol. Allocation of this budget could perhaps be placed in the hands of the National Health Committee and Pharmac may wish to take an arm's length role in this process. Pharmac would be free to raise or lower this 'tithe' as it saw fit. This way, the vast majority of Pharmac's budget would be based on utilitarian lines (perhaps with the Rawlsian wrinkle suggested above) while significant funds would be reserved for drugs meeting society's for non-utilitarian desires and preferences. But, again, Pharmac should not concern or distract itself with these considerations.

An alternative to this approach would see Government itself setting up a special fund for pharmaceuticals not deemed cost-effective by Pharmac, but it seems doubtful that this approach to dealing with RR would be nearly as straightforward as Pharmac acting directly.

4 Set a Maximum Price per QALY

I believe that Pharmac should set a firm ceiling on what it is prepared to pay in terms of dollars per QALY. Pharmac should make an initial determination of the maximum $/QALY value it can afford based on an analysis of currently funded drugs and existing CUA analyses. In general, Pharmac should expect to pay considerably less than this maximum, which would come into play primarily in the setting of high cost drugs.

The pharmaceutical products purchased (or subsidised) at a given maximum price per QALY (MPQ) are analogous to the surgeries and other treatments provided within the 'financially sustainable threshold' that is now routinely calculated in waiting list management. In this latter setting, the 'common currency' used to compare patients is a standardised priority score. As in this latter setting, Government would be free to add or subtract funds to Pharmac's budget in order to change the MPQ. As Prof Gillon says, if Government wishes to mandate that certain drugs be subsidised -- based on, say, RR – they should also foot the bill.

Pharmac's MPQ would likely fluctuate from year to year, based on budget, recently approved drugs, and a variety of other factors. In principle, the MPQ could serve as an index of public wealth, like gross domestic product. Government could be lobbied to provide necessary funds to raise the MPQ based on analyses of what additional drugs could be covered at different values of this measure. In this regard, the gap between currently affordable MPQ and what some might consider a more appropriate – higher – value, could at least be specified. Again this parallels the elective surgical arena, where the gap between financially sustainable thresholds and 'clinically desirable thresholds' (for provision of publicly funded surgery) are specified; in both cases the costs required to bridge the gap are calculable.

5 Coordinate PBMA with CUA

The principle of programme based marginal analysis (PBMA) is widely accepted in New Zealand health administration circles, although the extent and nature of PBMA implementation likely varies considerably. Not much was said in the background material about the methods currently used by Pharmac to determine which drugs to 'de-list' (or 'de-subsidise') when new drugs are subsidised whose cost would break the budget if other drugs were not de-listed. Presumably such decisions are based on CUA but whether and how candidates are identified for possible de-listing and confirmatory CUA is unknown to this writer.

One possible embellishment on whatever the current process is might be to encourage advocates of new drug funding to identify candidates for delisting, if such are needed to 'make room' for the desired new drug. This technique, while perhaps not particularly practical, rather neatly parallels the strategy used by Pharmac to enhance competition amongst pharmaceutical companies.

6 Calculating QALYs

The proposed focus on CUA and specification of an MPQ would draw new attention to the methods used by Pharmac to calculate QALYs. As long as Pharmac has nominally been using $/QALY as only one factor in a multi-factorial decision, Pharmac's avowed willingness to 'trump' CUA considerations (however rarely this actually happens) has probably distracted interested parties from the details of CUA – which however fascinating they may be to some are dry as dust to most people. The new approach, if implemented, would likely motivate pharmaceutical companies, doctors, and patient advocates to overcome any reticence to familiarise themselves with the various methodological considerations that attend the practices of estimating health outcomes and costs and assigning values to the outcomes.

The standardisation of values across different outcomes requires use of a generic measurement or valuation scheme onto which outcomes from many different kinds of treatments can be mapped (i.e., placed in accordance with the magnitude of desirability of those outcomes). There are two basic ways to develop such a scheme.

6.1 Simple Overall HRQOL Approach

The simplest valuation approach would be to use a 0 – 100 scale representing death to best possible HRQOL. The results of health outcome questionnaires, as reported in published studies, would be mapped onto this scale, based on the reported mean values of before and after health status. For example, suppose a sample of patients scored mean 3 on a five-point scale where zero the worst and 10 was the best. Suppose further that half of these patients took some drug and the other half took a placebo; suppose even further that after treatment the drug group scored mean 4 while the placebo group continued to score a mean of 3. Assuming this difference was statistically significant, the change of 1 level in 5 represents a 20 percent improvement, which on a 0 – 100 scale represents 0.20 QALYs (assuming the benefit last a full year - perhaps Pharmac should require that outcomes be reported no less than one year after initiation of treatment). Continuing this example, if Pharmac QALY Price was (say) $10,000, it would be willing to subsidise this drug at a maximum cost of $2,000 per year. (Often the actual price paid would be less due to negotiations and deal-makings of various kinds.)

6.2 Multi-attribute Utility Approach

A more complex but more nuanced approach to converting health outcomes into a common currency is to make use of a scheme of weighted health states, as explained in Pharmac's background material. That material describes how a descendent of the original Rosser-Kind Index called the Quality of Life and Health Questionnaire (QLHQ), was tested by Pharmac analysts for this purpose. Pharmac analysts (Mssrs Metcalfe and Sharplin) were able to map results from various other questionnaires onto the simple 4 x 4 matrix (levels of suffering vs. levels of limits on activities). Each of the 16 cells (e.g., mild suffering and moderate limits) was assigned a numerical weight based on empirical preferences derived from various kinds of people in the United States.

Pharmac abandoned its work with the QLHQ when it received negative feedback from external peer reviewers. The main objection was that the questionnaire was little known compared with those that have been used in most health outcome studies.[xi] This was true enough, although the QLHQ has been used in some health outcome studies (including advanced cancer patients[xii] and head injury patients[xiii] and there is no scientific reason why it could not have been used in many other studies. See Appendix C for further discussion of the QLHQ.

In my opinion, the greatest limitation of the QLHQ was not mentioned by critics, namely that the suffering dimension is limited to *physical* suffering, e.g., pain, nausea, shortness of breath. Psychological suffering (e.g., anxiety and depression) was not included due to concerns about over-inclusiveness in what was contained in the suffering dimension. However, further experience and reflection leads me to believe that the original approach used by Paul Kind and Rachel Rosser was more appropriate, i.e., a 4 x 7 level matrix of disability and 'distress', which included both physical and psychological distress or suffering. The 7th dimension of suffering was death, which (when weighted at zero) permits mortality or life-expectancy considerations to be factored into the QALY calculation. The absence of this capability in the QLHQ is also a considerable drawback. Finally, the QLHQ weights are more than ten years old and derive from the United States.

Accordingly, I recommend that Pharmac develop a refinement of the Rosser Kind Index, perhaps reducing the number of distress levels from 7 to 5 or 6 for ease of computation and outcome mapping. New Zealand values should be obtained for health state weights, perhaps using the kind of process described in the J Clin Epidemiol papers cited in Appendix C. A search for preference subgroups across demographic lines would be worthwhile, although whether Pharmac could or should develop separate subsidy schedules for different ethnicities, say, based on differences in preferences is perhaps doubtful. In any case, evidence to date shows that people think relatively alike in valuing pain and disability (and relief therefrom).

Once the matrix of weighted health states is in place, the results of health outcome studies (e.g., drug studies) could be used to map patients into the various cells of the before-and-after / treated-untreated matrices. For each drug and target population one set of before-and-after treatment matrices (e.g., 4 x 7 levels of disability and distress in the original Rosser Kind Index)) must be generated for both treatment and control groups. 'Difference scores' between before and after values would be calculated and compared across treated and control subjects, with the arithmetic difference between these difference scores constituting the net health gain produced by the treatment.[xiv]

The assignment of patients to the weighted cells in the matrices of disability x distress determines the value of the service. By multiplying the number of patients in each cell by the weight of that cell and then adding the resulting products, the value of the health service can be calculated. In this fashion, health outcomes can be mapped and compared relatively objectively and transparently.

Mortality considerations are much less frequently an issue than quality of life outcomes, but at times life-expectancy will be an issue, one way or another (usually to lengthen it, but not always). Methods exist to estimate the life-expectancy implications of mortality statistics, as commonly reported (e.g., the Decreasing approximation of life-expectancy method). The presence within the modified RKI of a 'dead' cell, weighted zero, should sufficiently accommodate mortality considerations, although some modeling work would be useful here.

The above described process represents the kind and level of objectivity tempered with public input that I believe will be required to withstand scrutiny of CUA results in the new era of prioritisation – as pioneered, perhaps, by Pharmac.

Before concluding, it is worth noting that CUA analyses are able to place the results of pharmaceutical trials into proper clinical perspective. So often the results are presented as (say) "a 20% decrease in mortality", which usually translates into something like a 4% death rate rather than a 5% death rate – often not even clinically significant, even if statistically significant. Such a one-percent reduction in absolute mortality risk (as opposed to the figure of a 20% *relative* risk reduction) implies that about 99 people would need to take the drug in order for one additional person to survive to whatever time point was assessed in the study.

Modeling and assumptions will need to be made throughout the CUA process, as always. It may be useful (and perhaps even necessary) to develop formal policies and procedures regarding how this will be done. A certain degree of subjectivity will be inevitable but can be substantially reduced when placed in an agreed framework. Sensitivity analyses can be conducted around any subjective areas in order to determine if further attention to these areas might be indicated.

Finally, the apparent formulaic or mechanistic nature of the proposed valuation process could make many people nervous, and perhaps rightly so. The 5% 'tithe' proposed above to fund non-cost-effective drugs might help in this regard, but a case could also be made that additional input regarding matrix cell assignment should be sought before assignments are finalised. This input could take the form of other analyses or information provided by medical or patient advocacy groups, or even testimonials from patients, family members, or health providers. Perhaps the receipt of such input could be an appropriate role for the kind of 'ethics committee' that Prof Gillon recommends. Decisions by Pharmac on mapping placement, in consideration of all evidence, would be final. Note again that what would be contestable is the number of patients assigned to the respective cells in each matrix – not the values assigned to those cells, as these stay fixed based on the empirical data obtained previously.

7 Appendix A Article in Today's *New York Times*: A Cancer Drug's Big Price Rise Is Cause for Concern

A Cancer Drug's Big Price Rise Is Cause for Concern

By ALEX BERENSON

Published: March 12, 2006

On Feb. 3, Joyce Elkins filled a prescription for a two-week supply of nitrogen mustard, a decades-old cancer drug used to treat a rare form of lymphoma. The cost was $77.50.

On Feb. 17, Ms. Elkins, a 64-year-old retiree who lives in Georgetown, Tex., returned to her pharmacy for a refill. This time, following a huge increase in the wholesale price of the drug, the cost was $548.01.

Ms. Elkins's insurance does not cover nitrogen mustard, which she must take for at least the next six months at a cost that will now total nearly $7,000. She and her husband, who works for the Texas Department of Transportation, are paying for the medicine by spending less on utilities and food, she said.

The medicine, also known as Mustargen, was developed more than 60 years ago and is among the oldest chemotherapy drugs. For decades, it has been blended into an ointment by pharmacists and used as a topical treatment for a cancer called cutaneous T-cell lymphoma, a form of cancer that mainly affects the skin.

Last August, Merck, which makes Mustargen, sold the rights to manufacture and market it and Cosmegen, another cancer drug, to Ovation Pharmaceuticals, a six-year-old company in Deerfield, Ill., that buys slow-selling medicines from big pharmaceutical companies.

The two drugs are used by fewer than 5,000 patients a year and had combined sales of about $1 million in 2004.

Now Ovation has raised the wholesale price of Mustargen roughly tenfold and that of Cosmegen even more, according to several pharmacists and patients.

Sean Nolan, vice president of commercial development for Ovation, said that the price increases were needed to invest in manufacturing facilities for the drugs. He said the company was petitioning insurers to obtain coverage for patients.

The increase has stunned doctors, who say it starkly illustrates two trends in the pharmaceutical industry: the soaring price of cancer medicines and the tendency for those prices to have little relation to the cost of developing or making the drugs.

Genentech, for example, has indicated it will effectively double the price of its colon cancer drug Avastin, to about $100,000, when Avastin's use is expanded to breast and lung cancer patients. As with Avastin, nothing about nitrogen mustard is changing but the price.

The increases have caused doctors to question Ovation's motive — and left lymphoma patients wondering how they will afford Mustargen, which is sometimes not covered by insurance, because the drug's label does not indicate that it

can be used as an ointment. When given intravenously to treat Hodgkin's disease, its other primary use, the drug is generally covered by insurance.

"Nitrogen mustard has been around forever," said Dr. Len Lichtenfeld, the deputy chief medical officer of the American Cancer Society. "There's nothing that I am aware of in the treatment environment that would explain an increase in the cost of the drug."

Dr. David H. Johnson, a Vanderbilt University oncologist who is a former president of the American Society of Clinical Oncology, said he had contacted Ovation to ask its reasons for raising Mustargen's price.

"I'd like to have some evidence from them that it actually costs them X amount, so that the pricing makes sense," Dr. Johnson said.

"It's unfortunate that a price adjustment had to occur," Mr. Nolan said. "Investment had not been made in these products for years."

Ovation, a privately held company, also needs the money to conduct research on several new drugs for rare diseases, Mr. Nolan said.

He acknowledged that Merck still made Mustargen and Cosmegen, an antibiotic that is used to treat a rare childhood kidney cancer, for Ovation. He said he was not sure when Ovation would begin producing the drugs, and a Merck spokesman said that Merck would continue to provide the drugs to Ovation as long as necessary.

But people who analyze drug pricing say they see the Mustargen situation as emblematic of an industry trend of basing drug prices on something other than the underlying costs. After years of defending high prices as necessary to cover the cost of research or production, industry executives increasingly point to the intrinsic value of their medicines as justification for prices.

Last year, in his book "A Call to Action," Henry A. McKinnell, the chairman of Pfizer, the world's largest drug company, wrote that drug prices were not driven by research spending or production costs.

"A number of factors go into the mix" of pricing, he wrote. "Those factors consider cost of business, competition, patent status, anticipated volume, and, most important, our estimation of the income generated by sales of the product."

In some drug categories, such as cholesterol-lowering treatments, many drugs compete, keeping prices relatively low. But when a medicine does not have a good substitute, its maker can charge almost any price. In 2003, Abbott Laboratories raised the price of Norvir, an AIDS drug introduced in 1996, from $54 to $265 a month. AIDS groups protested, but Abbott refused to rescind the increase.

And once a company sets a price, government agencies, private insurers and patients have little choice but to pay it. The Food & Drug Administration does not regulate prices, and Medicare is banned from considering price in deciding whether to cover treatments.

While private insurers can negotiate prices, they have limited leeway to exclude drugs from coverage based on price, said C. Lee Blansett, a partner at DaVinci Healthcare Partners, which works with drug makers on pricing and marketing.

"Price is simply not included in whether or not to cover a drug," Mr. Blansett said.

The result has been soaring prices for some drug classes, notably cancer treatments. In 1992, Bristol-Myers Squibb faced protests for its plans to charge $4,000 a year for Taxol, a breast cancer treatment.

Now, most new cancer treatments are priced at $25,000 to $50,000 annually. In some cases, companies are pushing through substantial price increases on already-expensive drugs.

Last year, Genentech raised the price of Tarceva, a lung-cancer drug, by about 30 percent, to $32,000 for a year's treatment.

In an interview last month, Dr. Susan Desmond-Hellmann, the president of product development for Genentech, said that the company had raised Tarceva's price because the drug works better than Genentech had anticipated.

"Tarceva was a more powerful and more active agent than what we understood at the time of launch, and so more valuable," she said. In an environment of soaring cancer drug costs, Mustargen's previous price was a comparative bargain, giving Ovation the opportunity to raise it substantially, said Dr. Richard Hoppe, a professor of radiation oncology at Stanford University and an expert in treating cutaneous lymphoma.

Mustargen's patent protection expired many years ago, so any company can make it. But because its sales are tiny, no drug maker has invested in a generic version.

"There's only one company that makes the drug, and they can decide what it's worth," Dr. Hoppe said.

Nitrogen mustard was initially tested as a chemical weapon. Its properties as an anti-cancer agent were discovered more than 60 years ago; today, it has been superseded by newer, less toxic medicines, and it is a niche product, with sales of only $546,000 in 2004, according to IMS Health, a market research firm.

Still, Dr. Hoppe and other oncologists call nitrogen mustard an effective treatment for cutaneous lymphoma, which initially appears as a rash but can turn deadly if it spreads inside the body. Some patients need only tiny amounts of the ointment, but others must apply it every day across large areas of their bodies.

For instance, Ms. Elkins has a severe case of lymphoma and must cover much of her body with Mustargen each day, a process that requires her to refill her prescription every two weeks. She said that the ointment was working, so she and her husband would find a way to pay for it.

Mr. Nolan of Ovation said that his company intended to work to improve access to insurance coverage for Mustargen. But Ovation has just begun to petition insurers to cover the drug. Meanwhile, patients are paying Mustargen's new, higher price out of pocket.

This is not the first time that Ovation has sharply raised the price of a drug it owns. In 2003, the company bought Panhematin, a treatment for a rare enzymatic disease called porphyria, from Abbott Laboratories. While Abbott still produces Panhematin, Ovation raised Panhematin's price, which had been $230 a dose, to $1,900, according to Desiree Lyon, executive director of the American Porphyria Foundation.

"It was a major increase," Ms. Lyon said. But she said that Ovation had worked to improve insurance coverage for Panhematin and to find ways for patients to get the drug even if they could not afford it.

Ovation also financially supports the porphyria foundation in its efforts to increase awareness of the disease and of Panhematin as a treatment, she said.

But many patients who rely on expensive drugs are stuck in a bind. Don Schare of Saratoga, Calif., said he paid $1,260 last month for 200 grams of nitrogen mustard cream, about 10 times what he paid for his prior prescription.

Mr. Schare, 69, said he was covered by the new Medicare Part D drug program and by supplemental insurance from AARP, but that neither of his plans covered Mustargen.

Jeffrey Malavasic, 58, a retired railroad worker in Florence, Ore., said he had decided to fill only half of his Mustargen prescription when he learned of the price increase. He used the drug sparingly in the past and will be even more frugal, he said.

8 Appendix B Synopsis of *New York Times* article: After Dreary '05, Drug Makers See Brighter Year Ahead

After Dreary '05, Drug Makers See Brighter Year Ahead

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Drug industry executives are voicing new hope that their companies are past the worst of the scientific, political and legal problems that dogged them through 2005.

After a long drought in finding new medicines, drug companies are filling their early-stage pipelines with promising new treatments, executives and analysts say. . . .

The most promising product in Merck's pipeline is Gardasil, a vaccine for cervical cancer that analysts say could become a multibillion-dollar seller. . . .

Sidney Taurel, the chief executive of Eli Lilly, noted several recent Food and Drug Administration approvals, including cancer drugs from Pfizer and Bayer; a rheumatoid arthritis treatment from Bristol-Myers; and Exubera, an inhaled insulin from Pfizer whose approval last week received attention as a significant new approach to controlling diabetes.
"There have been a number of product approvals which are showing the world that the industry has not lost its capacity to innovate," Mr. Taurel said. "There is a resurgence of productivity in research and development.". . .

Tony Butler, a senior industry analyst at Lehman Brothers, said he believed the industry might be through the worst of its crisis. "I am optimistic," Mr. Butler said. "I see light at the end of the tunnel, revenues improving off of a low base. And I see pipelines improving, midstage pipelines that I've never seen before."

The recent approval of cancer drugs like Sutent, from Pfizer, and Nexavar, from Bayer, has increased hopes that drug makers will be able to find new treatments as scientists unlock the pathways of disease at the cellular level, said Robert Hazlett, an analyst at SunTrust Robertson Humphrey. In the long run, Mr. Hazlett says, new drugs will be crucial to increasing sales and profit.

What we're seeing is that there is a glimmer of hope on the horizon and that these companies can return to growth post-2006," he said. "It's funny how some new drug approvals bolster the spirit of companies." . . .

9 Appendix C Development of Quality of Life and Health Questionnaire (QLHQ)

The QLHQ was developed to serve as a generic scheme onto which health outcomes of all kinds, measured by any validated instrument, could be reduced to a common currency of valuation. This development process was reported in several peer-reviewed publications (available on request). Many features of this process are relevant to the process proposed herein for Pharmac.

In 'The role of public values in setting health care priorities' (Social Science and Medicine 1991)[xv], I argued that public values should be incorporated into priority setting by way of values for *outcomes* as opposed to values for *programmes* (e.g., heart transplants vs. child care). This conclusion basically reinforced the concepts pioneered by Kind and Rosser.

In 'Multitrait-multimethod analysis of health related quality of life measures' (Med Care 1991),[xvi] Ron Hays and I showed that the clinical elements later incorporated into the QLHQ could be reliably assessed *and discriminated* using separate, independent methods of evaluation. The QLHQ is the only questionnaire to have been subjected to this form of testing; most have looked only at the statistical correlations across different items within the same questionnaire. For analytic reasons, such internal cross-checking is insufficient to establish construct (i.e., convergent and discriminant) validity (see Campbell and Fiske). This finding also supports the use of similar two-construct approaches to measuring HRQOL, including the proposed modified Rosser-Kind Index.

In 'Improving task comprehension in the measurement of health state preferences' (J Clin Epidemiol 1992),[xvii] my colleagues and I showed that a paired comparison task (similar to Point Wizard) produced somewhat more reliable estimates of health state preferences than did a direct rating task. Also, informational cartoon figures depicting various levels and combinations of suffering and disability were found to improve comprehension of the rating task. Such figures can also be useful for standardising responses across ethnic or cultural lines (as discussed in that article).

In 'Large-scale outcome evaluation: How should quality of life be measured? Part I' (J Clin Epidemiol 1995),[xviii] my colleagues and I described the process of assigning public values to the various combinations of suffering and limits on activities embodies in the QLHQ. A search for preference subgroups (i.e., subgroups like old and young or male and female whose preferences regarding suffering and disability may, in theory, differ from each other) was unrevealing.

Finally, in Part II of this article my colleagues and I described the use and further validation of the QLHQ in a cohort of patients with advanced cancer.[xix]

Taken as a whole, I believe (with acknowledged bias) that the QLHQ has a better scientific pedigree than most other generic health outcomes questionnaires. However, as noted in the text, the QLHQ has several flaws and I do not advocate its use. A more appropriate approach would be to develop a new instrument based on the Rosser Kind Index, as also discussed in the text. The theoretical and empirical support for the QLHQ, just described, can be expected to apply equally well to this new instrument, as the principles underlying its development would be identical to those used for the QLHQ.

10 Endnotes

[i] Berenson A. New A Cancer Drug's Big Price Rise Is Cause for Concern. *New York Times* 12 March 2006, http://www.nytimes.com/2006/03/12/business/12price.html?_r=1&th=&oref=slogin&emc=th&pagewanted=all

[ii] Electing to disregard this factor is also desirable from a methodological perspective, thus killing two birds with one stone.

[iii] A. Jonsen. Bentham in a box: technology assessment and health care allocation. *Law, Medicine and Health Care* 1986; 14: 172-174

[iv]

[v] Hadorn DC. Setting health care priorities in Oregon: Cost-effectiveness meets the Rule of Rescue. *JAMA* 1991; 265: 2218-2225.

[vi] L. Trachtman. Why tolerate the statistical victim? *Hastings Center Report* 1985; 15: 14.

[vii] Ibid., p. 171.

[viii] A. Gibbard, The prospective pareto principle and equity of access to health care. The President's Commission for the Study of Ethical Problems in Medicine and Biomedical and Behavioral Research. 1983. Securing Access to Health Care: The Ethical Implications of Differences in the Availability of Health Services. Volumes 2. Washington, DC: U.S. Government Printing Office, 1983 (Henceforth referred to as the "President's Commission-Securing Access."), pp. 153-178 at p. 177.

[ix] Supra note *iii*.

[x] A. Gibbard, supra note *viii*, at p.177.

[xi] Criticisms were also received by Pharmac regarding the QLHQ on other grounds, including that it does not permit assessment of states worse than death and that its weights were not derived from risk-based measures, as required by contemporary neo-classical economists. Neither of these issues seems particularly relevant in this context.

[xii] Hadorn DC, Sorenson J, Holte J. Large-scale outcome evaluation: How should quality of life be measured? II: Questionnaire validation in a cohort of patients with advanced cancer. *J Clin Epidemiol* 1995; 48: 619-629.

[xiii] Steadman-Pare D, Colantonio, A; Ratcliff, G et al. Factors associated with perceived quality of life many years after traumatic brain injury. *J Head Trauma Rehab*.2001; 16:330-342.

[xiv] Standard deviations around the mean scores would be required in order to specify the distributions of patients across cells. These are almost always provided in study reports. A bivariate normal distribution would be assumed (i.e., levels of distress and disability both normally distributed) during the mapping process.

[xv] Hadorn DC. The role of public values in setting health care priorities. *Soc Sci Med* 1991; 32: 773-782.

[xvi] Hadorn DC, Hays RD. Multitrait-multimethod analysis of health related quality of life measures. *Med Care* 1991; 29: 829-840.

[xvii] Hadorn DC, Hays RD, Uebersax J, Hauber T. Improving task comprehension in the measurement of health state preferences: A trial of informational cartoon figures and a paired comparison task. *J Clin Epidemiol* 1992; 45: 233-243.

[xviii] Hadorn DC, Uebersax J. Large-scale outcome evaluation: How should quality of life be measured? I: Calibration of a brief questionnaire and a search for preference subgroups. *J Clin Epidemiol* 1995; 48: 607-618.

[xix] Hadorn et al. 1995; supra note *xii*.

PHARMAC and the funding of high-cost pharmaceuticals

George Laking

1 Author's Perspective

I am a medical doctor with a research interest in the economics of diagnosis, which I have applied practically in health technology assessment (HTAs) of medical diagnostics. The methods I use emphasise cost-utility analysis (CUA) and the statistical handling of uncertainty. When not working on economics I also do some clinics and research in medical oncology. Although I have not personally been involved in the kind of decisions made by PHARMAC, I have followed these with interest, particularly the technical use of CUA. My prior standpoint on funding was pretty much that of maximising QALYs. The background reading and the reports have certainly made me more sensitive to the range of moral positions.

2 Comments on the reports

Hansen is an economist from the University of Otago who has developed a computerised tool for multi-criteria decision making. Gillon is Emeritus Professor of Medical Ethics at Imperial College London and has written a standard text on his subject. Both authors have given considered, constructive responses to PHARMAC's questions. Gillon's work includes a reply to Hansen, but not vice-versa. I think it would be worth offering the authors at least another round of exchange. Both writers recommend that PHARMAC change its approach to decision making on high cost pharmaceuticals (HCPs). Although he is appropriately diffdent, Hansen would like PHARMAC to use his computer-based multicriteria decision software. Gillon would like PHARMAC to create a new advisory committee on allocation, on a par with PTAC but in the domain of social value.

2.1 Hansen

Hansen is motivated by the idea that PHARMAC should 'be more explicit and transparent' in its decision-making, and that to do this requires the use of 'technical methods'. CUA offers the technical means to describe changing costs and effects across different treatments in terms of a 'health possibilities frontier'. In figures taken from his earlier Treasury working paper, Hansen shows how various theories of distributive justice map to different points on the health possibilities frontier. This illustrates PHARMAC's finding that 'CUA [alone] cannot explicitly assist in any debate about the ethics of maximising health' (Anonymous 2004). The challenge for decision makers presented with CUA is to identify

tradeoffs between patient groups that are deemed 'equitable' or 'distributionally just'.

Hansen argues that 'there is a tendency in decision-making situations . . . for criteria that are based on so-called "hard" data . . . to overwhelm other criteria'. He suggests that this tendency may inadvertently bias decisions towards Benthamist utilitarianism. His proposed remedy is to fortify the technical means of handling value judgments. Here Hansen identifies the 'four step approach' of the Oxford Priorities Forum (Hope Reynolds and Griffths) as a starting point. Within this he recommends PHARMAC 'tighten' (i.e., specify more precisely) its decisionmaking criteria, in consultation with its constituencies. Finally, he recommends that PHARMAC consider using multi-criteria decision analysis. He declares an interest in his own computer package, 'Point*Wizard'. I will return to this below.

In summary, were Hansen's recommendations to be adopted, PHARMAC's Operating Policies and Procedures would perhaps be rewritten in Section 2 to give more detail on decision criteria, and Section 4 to describe the new technical means of handling distributional issues.

2.2 Gillon

Gillon's recurring point is that 'when agreed moral principles or values come into conflict judgement is required and unfortunately the moral approaches to carrying out such judgement are disputed'. He recommends against 'building into PHARMAC's procedures a morally contentious mathematical, computerbased, approach to moral judgement that is likely to be vigorously, vociferously, and conscientiously rejected by many'. Instead PHARMAC should 'stick with . . . a variant of implicit judgement when moral values conflict, while making explicit the moral values considered to be relevant and in conflict'.

To facilitate this Gillon recommends creation of an 'allocation committee' composed of 'conscientious people drawn from a variety of perspectives'. This committee would 'draw on such models as a clinical ethics committee, the Oxford Priorities Forum, and the nice Citizens Council. He describes its role as advisory, which suggests that it would stand outside PHARMAC's main decision-making structure. He also recommends that PHARMAC specify an explicit ethical framework within which to make allocation decisions, offering Beauchamp and Childress's formulation (autonomy, non-malevolence, beneficence, and justice), and UNESCO's Declaration on Bioethics and Human Rights as possible alternatives. Finally Gillon recommends that PHARMAC's appeal mechanisms be more clearly specified, in the pattern of Daniels and Sabin's 'accountability for reasonableness'. He has not however suggested a specific alternative to the current possibilities for informal appeals to Government via public and media opinion and a formal appeal process through the courts.

Were Gillon's recommendations to be adopted, PHARMAC's Operating Policies and Procedures would perhaps be rewritten in Section 1 to introduce the Allocations Committee (on a par with PTAC) and state the ethics framework (maybe after the paragraph on the Treaty of Waitangi). Overall, Gillon's recommendations are broadly compatible with those of Hansen, if only because they are pitched at a different level of PHARMAC's operation.

2.3 A Synthesis

As I have suggested above, the basic difference between the two authors is that Hansen defends technical methods while Gillon defends judgement. Gillon has elaborated his thinking in this respect further than Hansen (noting that Hansen was not writing a reply). For example Hansen dismisses 'nontechnical' approaches in just a line, saying that they 'tend to rely on political expediency and lobbying by interest groups and ad hoc decision-making that often favours the status quo'. Gillon by contrast is positive about the political process, describing PHARMAC as 'respecting the autonomy of the people of New Zealand collectively, to the extent that democratically elected governments respect such autonomy'. Gillon defends 'vigorous appeal, often with media amplification . . . and . . ."shroud-waving"[as] valid components of both the democratic process and of an acceptable substantive theory of justice'.

I think few people would want all pharmaceutical funding to be decided in 'trial by media' or trial by court. It could be helpful if Hansen or a similarly-minded person were to develop the distinction between technical and non-technical approaches in a way that engages with rather than rejects political expediencies. Technical approaches to pharmaceutical funding still need to be politically palatable.

Hansen has suggested that his computer program Point*Wizard be used as a technical means to resolve value judgements in pharmaceutical allocation. I think Gillon is right to recommend caution. Although there is experience with points systems in other areas of life, Hansen has not given an example of their use to decide the across-the-board funding of HCPs. None of the literature he cites has 'critical review' or 'systematic review' in its title. Hadorn found that points systems were 'as a rule, more accurate than human predictors' for medical diagnosis, but these data must be of limited applicability to the just allocation of pharmaceuticals, given the lack of a 'gold standard' for justice. These things all lead me to view the use of a points system in the present setting as experimental.

Hansen's account of points would benefit from further development more generally. For example he says that the systems have been 'near-universally found to out-perform purely intuitive decision-making approaches'. Yet he also

says that 'points systems are not a replacement for human decision making'. I would like him to clarify the dimensions on which point systems out-perform intuition. I would like him to clarify the salient features of settings in which point systems would simply attenuate the need for implicit judgement (in the parlance of our times, features that tell us when a decision is a 'no-brainer'). I would certainly hope to see these kinds of issues addressed in any more formal research proposal.

3 Some Comments on Technical Approaches

I will finish with some thoughts of my own on the relative roles of technical approaches and moral judgement in deciding pharmaceutical funding. Hansen has identified explicitness, transparency, and consistency as the aims of technical approaches to decision making. Strictly speaking these are 'process aims' that relate to the way in which decisions are made, in contrast to the ultimate aim of making the right decisions.

These aims head up a longer list of what I see as virtues of technical approaches, and to which I would like to add at least three more items. First, there is a virtue of procedural effciency which follows from having worked out in advance a method that can be applied in a timely way to real-world decisions by appropriately trained workers. Although good judgement is important, it takes time, and technical approaches may offer workable interim solutions while philosophers deliberate.

Secondly, there is a reputation for procedural impartiality that derives from use of methods external to the decision-maker or analyst's psyche. Although this reputation is in many ways a consequence of explicitness and transparency, it has value in its own right in any public institution. It means that workers can be trusted to divorce the application of the method from their own self-interest.

Closely related to procedural impartiality is a virtue of dispassionate implementation. Although I agree that 'all approaches to deciding which pharmaceuticals to fund, including high cost ones, are inherently normative', it is worth distinguishing between an approach and the instruments it uses. In technical approaches, little extra normative content is introduced after the initial design of the instruments. Therefore much of the attraction of technical approaches is to take the moral heat out of decisions about health funding[1]. The

1An example with a little too much moral heat is Williams' 'story of Jim and Pedro', said to demonstrate the 'moral contentiousness of the mathematical approach to moral judgement' (Williams 1973). Pedro offers Jim the botanist the 'privilege' of shooting an indigenous Indian pour encourager les autres, but if Jim declines, Pedro will shoot twenty. It is clear that Pedro does not like Indians; it is not as clear why he wants to bring mathematicians into disrepute. Although I agree that this is one possible argument against an ethic of maximising utility, I cannot see that it is a general argument against the use of mathematics in moral reasoning. For example, we can change the numbers so that Pedro will shoot only one Indian. However let us

approach is normative, in that it entails values, but the overarching values and the freedom of moral action are determined elsewhere. In the case of a government agency such as PHARMAC, elsewhere means the democratic institutions making public law.

To me personally the scope of morality to decide the funding of the New Zealand Pharmaceutical Schedule seems a little constrained, so long as the country continues to restrict eligibility mainly to its own nationals. The moral arguments that make most sense to me are those that might be used in deciding claims policies for an insurance scheme (on reflection this is probably still only a minor constraint on the scope for morality). One subject on which I have found accounts of distributive justice in health care to be relatively quiet is how to get it wrong. Not only does fair allocation of resources pose an intractable problem, it must be decided under severe constraint of time. Moral error is inevitable – one of the more egregious examples being the death of the child Coby Howard from leukaemia in a window of time between reversals of decisions on state funding of bone marrow treatment in Oregon (Hadorn 1991).

Moral thinkers of all grades have given advice on this subject, from Luther ('as long as we are here . . . we have to sin') to Lenin ('if you are going to make an omelette you have to break eggs'). Here at least Benthamist utilitarianism could both justify a practical mode of action (maximise QALYs), and absolve the agent from responsibility for error (transmuted into decision uncertainty or QALY tradeoffs).

I will finish by suggesting a mode of action that could accommodate both Hansen and Gillon's recommendations with those of an unreconstructed costper-QALY optimiser. The QALY optimiser's preferences are strongest at the extremes of cost-effectiveness; dominant and low cost-per-QALY interventions are strongly 'in', and dominated and high cost-per-QALY interventions are strongly 'out'. The QALY optimiser is maximally indifferent about cases at the margin. It does not really matter economically if these cases are in or out. Yet it is the cases at the margin that matter most in human terms, because these are the people who lose, having almost won, or who win, having almost lost (Metcalfe, Dougherty, Brougham and Moodie 2003).

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say that Pedro has neurosyphilis, cannot aim straight, and his shot guarantees an agonising death, while Jim is a former US Marine sniper who kills instantly. Now the choice for Jim is similarly odious, but this time it is qualitative and not mathematical. My position on mathematics is that it is simply amoral, a dispassionate, value-free instrument that can be brought in to assist with some forms of reasoning. Although it is hard to see how the kind of mathematics used in deciding health care allocation could help Jim or the Indians, no one would criticise a screwdriver for failing to disarm a nuclear warhead. Issues of health care allocation are a small and relatively genteel subset of all moral possibility. The story of Jim and Pedro does not rule out the existence of defined areas of the moral landscape within which mathematics might be a wholly proper tool to use.

Therefore apart from the philosophical and ethical oversight of the process as a whole, it seems there is a special need for this expertise to scrutinise the margins. But there are neither enough philosophers, ethicists, or time to cover everything. Here multicriteria decision analysis could screen for conflicts of values, in practice those cases where the cost-per-QALY is high but there are important competing considerations. In some cases, multicriteria methods might clarify things enough for a decision to be made. Otherwise the case could be referred to the allocation committee. I would like to see the allocation committee itself working within a fiscally neutral framework such as PBMA. The requirement for fiscal neutrality is one of the admirable things about PHARMAC, in contrast to other agencies such as NICE.

Manchester, United Kingdom, February 2006

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DECISION-MAKING FOR HIGH COST PHARMACEUTICALS.

Comments by Robert Logan on reports from Prof R Gillon and Dr P Hansen

The Board of Pharmac are to be congratulated on taking a systematic approach towards funding decisions for high cost pharmaceuticals. As will be discussed, the pressures on Pharmac are likely to accelerate with technological advances, rising consumer expectations and demands in an increasingly pluralistic society.

After commenting briefly on the Hansen and Gillon reports I shall highlight some relevant contextual issues before concluding with suggestions for further steps Pharmac may wish to consider.

The Gillon and Hansen reports

The reports are interesting, informative and complementary. They highlight the moral significance attributed to health inequalities. In identifying the need to enhance Pharmac's decision-making processes, both emphasize the dangers of basing moral judgements on quantitative data with Gillon giving explicit warnings about being seduced by fancy formulae and measurements such as QALYS and CUA. Nevertheless the quantitative approach advocated by Hansen clearly provides a most useful aid. They agree on the need for greater clarity and specificity of Pharmac's decision-making criteria with Gillon drawing attention to the importance of specifying the meanings of key words such as "needs", "benefits" and "autonomy". It would perhaps have been relevant to his discussion on the important subject of autonomy to have made the point that rights and autonomy are more than "I must have" and carry with them the generally ignored obligations to respect and respond to the rights and independence of others [J Locke & J S Mills].

They agree that the processes for funding decisions for high cost pharmaceuticals should be an extension of that applied to lower cost drugs.

Whilst both refer to the influence of normative factors on decision-making, neither addresses the New Zealand context, and in particular the philosophies and approaches to decision-making of Maori and Pacific Islanders which differ from those of Europeans.

Neither comments on the need for flexibility of decision-making processes in the face of changes in pharmaceuticals and health service delivery. The four-stepped process advocated by Hansen and supported by Gillon makes a lot of sense as does his suggestion that it is examined as a concomitant research project. Suggestions for the establishment of an allocation advisory committee and an explicit appeal process merit serious consideration.

Gillon emphasizes that in its preoccupation with responding to the demands for new funding Pharmac should not lose sight of the need to ensure the most appropriate use and distribution of current scheduled drugs. The linkages between decision-making and source of funding are not addressed by either commentator. It is my understanding this comes from DHBs rather than Pharmac or government. Surely this has relevance as to who takes responsibility for difficult decisions involving high cost pharmaceuticals and, as Gillon highlights, raises questions about the government's obligation to fund any extra subsidies it instructs Pharmac to provide.

A key issue is how the actual decisions are made. The commentators express uncertainty about the ways in which Pharmac interprets current decision criteria. There is also a lack of clarity about how these criteria are used and the means by which the Board reaches its final decisions - ? by vote, ? true consensus, ? consensus led by a strong individual etc.

Some contextual issues

Despite emphasizing the importance of normative factors in decision-making neither commentator actually examines the New Zealand context which Pharmac needs to understand as background for its decisions.

New Zealand is not a particularly wealthy country situated in the South Pacific with historical and statutory obligations to Maori. Beyond recognizing and responding to the implications of the Principles of the Treaty of Waitangi, Pharmac, in its ethical considerations, needs to consider the impact of the whanau focus of Maori ("we" rather than "I"). This may have greater relevance to funding decisions than the individualist ethos of European philosophy.

The general expectations of health care and of Pharmac in particular, have risen, particularly among the elderly and their doctors and carers compared with as recently as 20 years ago. Society is also becoming more ethically pluralist reflecting in part immigration of people from Pacific Islands and Asia bringing with it non-European ethics and attitudes. These invariably impact on expectations for health care. For example, the wide variation in attitudes to "rescue" treatment can, at one extreme, result in quite inappropriate demands.

The health sector also constantly changes. In the current phase of decentralization DHBs are taking greater responsibility for decisions on the adoption of new technologies. Cynically one might wonder whether the establishment of Funding and Planning Departments has increased the opportunities for passing "unpalatable" financial parcels! Clinicians as well as managers and funders are now recognizing the need for formal prioritization of competing demands. With the government's desire to curtail spending in the health sector such awareness is likely to increase so that the challenges confronting Pharmac may be better appreciated by its critics! The predicted workforce shortages in the face of accelerating demand will almost certainly have implications for the types of medication prescribed and how they are administered.

There will never be sufficient resources, financial or otherwise, to meet the health expectations of the community. Surprisingly, there seems to be a lack of awareness or perhaps denial that potential consumers and their clinicians are competing with one

another for funding. The speed of technological advancement is increasingly outstripping changes to the health sector structure and function. Such divergence should promote questions for Pharmac to consider - "by whom?", "to whom?", "when and how treatments are given". Pharmac's current role is unclear: is it an agent for the DHBs, a referee between competitors for funding, a "dictator" or leader for quality use of medicines etc.

The report from the National Health Committee on decision-making about new health interventions [May 2005] highlights the many complex factors in addition to the quantitative, scientific or financial which influence decisions.

As encapsulated in Gillon's quote from Immanuel Kant , decision making in the face of competing values and interests cannot be resolved but only aided by quantitative measures – there will always be considerable uncertainty surrounding the process.

Recommendations for Pharmac to consider

On the basis of the two reports and the thinking which they have stimulated, I offer the following recommendations for Pharmac to consider:

Principal recommendations

- It must be made clear that the quantitative assessments and comparisons which form the basis for decision-making are value free and are quite separate from that part of the process.

- In identifying key contextual factors the practical implications of the Treaty of Waitangi and relevance of non-European ethical perspectives to both process and content must be acknowledged and explored, particularly in relation to health disparities.

- The fairness of the decision-making process requires clarification of its explicit and implicit components. Key words such as "best health outcomes", "effectiveness", "needs", "benefits", "autonomy" etc should be defined. Whilst the final steps may be implicit, Pharmac can probably be more open if it understands how decisions are actually reached by the Board.

- Serious consideration should be given to establishment of an advisory committee whose composition reflects significant cultural perspectives within the population, particularly Maori. The process for appointment of its members, terms of reference and ethical framework will need particular thought and care. Consideration of the history, composition and function of the National Health Committee, an independent advisory committee, may be helpful. The advisory committee could act as "referee" in a "fair" appeal process.

Additional recommendations

- Pharmac must be involved early in national and regional planning for new clinical interventions, (eg cardiac stenting and organ transplantation) which may have long-term requirements for complex and expensive drugs.

- The criteria for assessment of preventive treatments should be reviewed in the light of frequent emotional pressures and discounting formulae which bias against them in favour of drugs with immediate or "life saving" benefits.

- Ways of promoting ongoing debate on prioritization to expose clinicians to the sorts of challenges confronting Pharmac merit exploration. Most work in clinical silos and as a result either fail to appreciate the demands of other specialties or have no opportunity to challenge them.

- The concomitant research project into the decision-making model proposed by Hansen and supported by Gillon will increase transparency and stimulate better understanding of the decision-making process.

- It may be appropriate for Pharmac to review its relationships with key players such as government, Ministry of Health and DHBs. The question surely needs to be asked of DHBs, both individually and collectively, as to whether they should be taking some responsibility for what are often contentious decisions rather than leaving them to Pharmac. They should surely be able to determine the funding of low cost/effective drugs, freeing Pharmac to deal more rigorously with controversial, complex or expensive claims.

- Clearer distinction is needed between the supply and demand aspects of Pharmac's work in statements such as its operating policies and procedure document.

On the basis of these excellent commentaries and their critiques Pharmac should be able to initiate steps to increase the appropriateness and fairness of its funding of high cost pharmaceuticals.

R L Logan
February 2006

COMMENTARY ON REPORTS BY PAUL HANSEN AND RAANAN GILLON ON PHARMAC'S APPROACH TO DECIDING WHICH HIGH COST PHARMACEUTICALS TO FUND

General remarks on both reports

Points of agreement

The most important and obvious point concerns the very high level of agreement between the two experts from different disciplines (namely, Paul Hansen from economics and Raanan Gillon from medical ethics) on the very difficult issues raised in the questions posed to them by Pharmac. Essentially, both agree that real world decisions on the use of scarce resources (in this case for pharmaceuticals) can rarely, if ever, be taken on the basis of a single, simple principle of social justice, that a wide range of principles of social justice find ethical and practical support in some quarters, and that there are inevitable conflicts between these different theories of social justice which organisations like Pharmac have to manage in pluralist, democratic societies through the exercise of judgement. Thus Hansen and Gillon agree that utilitarian health benefit maximisation such as through using cost-utility analysis (CUA) alone to determine decisions, is only one possible way of interpreting the imperative of 'maximising value for money' and may not be the most acceptable (indeed, they rightly imply that it is highly unlikely to be acceptable to most people most of the time as the single allocative criterion).

Though using different language, both further agree that there is a range of economically efficient distributions of Pharmac's resources between pharmaceuticals and patients. Hansen posits a 'health possibilities (efficiency) frontier' while Gillon talks about the production of a 'sufficiency of beneficial outcomes in the use of scarce resources', both of which admit to a range of socially just allocations. Normative decisions have to be taken, for example, to decide how the potential benefits accruing to different sorts of patients should be compared. Implicitly, both authors also agree that the value judgements taken by Pharmac (i.e. in terms of which distributional criteria to take into account and how much weight to give to each) may change over time as government, stakeholder and public opinions change. Both agree that Pharmac should make explicit (and in Hansen's case expressed more clearly than at present) which moral values it considers relevant and in conflict (though they disagree about how to bring these values to bear on decisions). Finally, they agree that Pharmac can expect a degree of conflict and dissatisfaction with whichever decision criteria are adopted and whichever pattern of decisions is taken since beyond the avoidance of 'waste' and 'inefficiency', there is little prospect of unanimity on the distributional principles required to determine how 'best' to allocate Pharmac's budget. I agree with their areas of agreement.

Differences of view

The principal apparent disagreement between Hansen and Gillon concerns the use of what Hansen terms 'Multi-Criteria Decision Analysis' (MCDA); i.e. the use of formal methods to determine explicit trade-offs between decision criteria and aggregating them to help decide which drugs to fund. Gillon is attracted to the simplicity and clarity of this 'mathematical approach', but ultimately argues against its adoption by Pharmac on the grounds that while it may be morally relevant to some decisions about conflicting moral

values, there will be other situations where the mathematical approach simply prejudges what are and should be unique decisions informed by conscience and ultimately subjective. Gillon takes to heart Kant's view that no single rule of judgement can be used to arbitrate between conflicting moral values or rules. Ultimately, the approach has to be case and context-specific.

My interpretation of the two reports is that the difference between the two authors is more apparent than real since Hansen only advocates the use of MCDA to 'support' decision makers and to 'help' decide which pharmaceuticals to fund, not to determine which to fund. Thus he implicitly allows space for the sorts of unique, context-specific judgements which Gillon believes are inescapable for 'just' allocation decision making. Hansen does not describe the circumstances in which Pharmac might decide to go beyond the results of a MCDA, but presumably this option would be available at all times, depending on the judgement of the Board (or whichever committee is charged with making the funding decisions). The fact that Hansen quotes approvingly from writers who conclude that MCDA is generally superior to more implicit, intuitive decision making processes suggests that he believes that MCDA should be used in the vast majority of cases, that there should be clear reasons for over-ruling its results and that its use should in general 'improve' the quality of Pharmac's decision making. This begs the question which Hansen does not answer as to what yardstick of 'improvement' or 'accuracy' (p22) of decision making he might have in mind.

In contrast, Gillon is far more sceptical about how to derive the empirical basis for MCDA (e.g. how to derive the weights to be given to each distributional criterion) and whether it is genuinely likely to be 'morally relevant' (p14) to specific decisions where moral values conflict.

Another consideration which may lie behind this (apparent) difference between the two authors is whether or not formal, explicit, numerical information has a tendency to 'trump' more subjective, intuitive judgements in real world group decision making and whether this leads to inappropriate, unjustifiable decisions in particular cases. Despite supporting MCDA, Hansen raises this possibility in his remarks about the use of cost per QALY analysis (p 15), but does not provide any evidence to back up this assertion. It might be useful to see what the empirical evidence on group decision making says on this issue. Hansen's antidote to any tendency for seeming 'hard' evidence to overwhelm other relevant evidence and thereby bias decision making is to specify decision criteria, including qualitative criteria, as precisely as possible (p16).

Another apparent difference between the two reports relates to appeals against Pharmac decisions. Gillon explicitly recommends that in addition to the courts and the 'court of public and political opinion', there should be a specified appeal process such as recommended by Daniels and Sabin as part of their 'accountability for reasonableness' procedures. In principle, this is a good thing, though there is the risk that it increases costs and workload for Pharmac, distracts scarce staff resources from more valuable activities and is used by interest groups to deter Pharmac from taking decisions in future which might go against their interests. If there were to be some appeals process short of litigation, then Pharmac would need to record the reasons and steps in each of its

decisions in a fair amount of detail. Again, this would probably be a good thing, with cost implications.

Neither Hansen nor Gillon explicitly recommends Pharmac recording and publishing the basis of their decisions, though Hansen would clearly be broadly favourable to such a procedure. It would seem to me to be a good thing, particularly if Pharmac chooses to retain a multi-criterion approach which includes scope for complex, case-specific, implicit decision processes. It should be possible after the event to describe what took place and to summarise the basis for the decisions reached, however 'messy'.

Comments on the Hansen report

This is excellent and very clearly written given the complexity of the subject matter. I particularly appreciated Paul's insistence that CUA is not intrinsically bound up with a utilitarian calculus and his use of the notion of an efficiency frontier which includes an infinite number of efficient but different distributions of resources as a way of clarifying thinking. I hope both will help shape Pharmac's policy development in this difficult area.

It seems to me that the report very clearly and sensibly takes Pharmac to the next stage if it wishes to refine and make more explicit its decision making processes by setting out broadly what the procedures need to be able to accommodate (i.e. some way of putting relative weights on an agreed set of decision criteria and of incorporating these weighted criteria consistently and transparently into decision making). Paul does not set out how this should be done, except to suggest that some form of what he calls 'multi-criteria decision making' should be adopted in support of decision making, but not as a replacement for other forms of judgement.

It seems to me that if Pharmac decided to develop such an approach (almost irrespective of the extent to which it was likely to use the MCDA results to determine decisions as opposed to simply informing them), Pharmac would need to determine what its preferred set of decision criteria are and the weights to be given to each. There are many ways of doing this from the Board doing it internally through to a very large scale, representative public consultation and all sorts of intermediate options costing less money and time. Ultimately, of course, the Board has to be happy that the criteria and weights are appropriate to NZ, workable, appropriate to Pharmac's remit and broadly consistent (or at least not to obviously at variance) with either broad government goals and/or other decision criteria used in the public health system. In practical terms, it might be a good idea to secure input from experts in consensus development methods (Delphi, nominal groups, etc) to help with this next stage. There is an expert team here at LSHTM with extensive experience in developing and using consensus methods, albeit mostly in relation to clinical guidelines, but directly relevant to what I sense you need next (Professor Nick Black would be the best first contact). The composition of any groups involved in reaching a consensus on decision criteria and weights is obviously going to be crucial to the outcome but also how the process is perceived. Your local knowledge will guide you as to which 'constituencies' you will need to involve, but this will bear careful thought. Paul presents 'multi-criteria decision making' as a decision aid not a replacement for what he calls 'human' decision making (i.e. more implicit processes). You need to be

aware that not all decision analysts share this view. Experts such as Jack Dowie (also as it happens at LSHTM and a New Zealander by birth!) take a much 'harder' line arguing that all values used implicitly by decision makers and their weights can and should be identified, quantified and modelled, and that the results should determine decisions since they must be superior to the results of other approaches. The Board may wish to decide whether a rigorously constructed decision analytic approach would not be consistently superior to implicit group methods. If you accept Paul's arguments at the top of page 22 about the superiority of criterion driven decision making, then there is an argument that it should always be preferred to other methods. I'm not advocating the hard or soft use of decision analysis, but do think it needs to be considered. My personal view is that the reality of a public body in a pluralist democratic society such as New Zealand's means that using some form of MCDA to determine decisions, however sophisticated, will always lead to the criticism of excessive reductionism and arbitrariness in decision making.

I do feel that this part of the report is a little vague as to precisely how and in which circumstances points systems 'out-perform' implicit methods. Is this mainly in terms of consistency? It is hard to see how one or other approach could be argued to be more 'valid' or 'accurate' since the more informal approaches will be taking a different range of factors into account with different weights at each decision while the MCDA approaches will inevitably be more consistent (from time to time possibly at the expense of making 'wrong' decisions). There is also the problem with studies comparing so called explicit with so called implicit approaches as to what the explicit approaches are being compared with since there is a spectrum of more and less implicit approaches. Pharmac generally would be at the more explicit end of the implicit spectrum, I would argue! It is also important to remember that there is no guarantee that using more explicit, transparent, consistent methods of decision making will reduce the level of criticism of individual Pharmac decisions or even criticism of the organisation as a whole, its role in the system, etc. I'm not sure whether there is much evidence either way on this. This will partly depend on the level of legitimacy with which Pharmac and its role is regarded by the public, patients, doctors, etc.

One final point about the weights given to the different decision criteria is that these are likely to change over time, assuming that they are derived, at least in part, from population valuations. This is for no other reason than the fact that the composition of the population is changing (e.g. through immigration). So, as the population ages, you might expect that the relative weight given to the predominant age group of the patients benefiting from a drug will change, perhaps in the direction of giving a higher value to older beneficiaries. This suggests that deriving criteria and weights is not a once and for all exercise but will have to reviewed from time to time.

I have a few much more minor points. I think the description of 'non-technical' decision making on page 4 is a somewhat pejorative description of more implicit processes. Also it seems to me to be just as much a political decision to use so called 'technical' approaches as to use so called 'non-technical' approaches!

My final minor point is to observe that while I agree that Paul's focus is not procedural, and that what he recommends is consistent with a range of processes, I do think that 'multi-criteria decision making' as he describes it, has major implications for the sorts of

procedures which Pharmac will have to follow (e.g. each decision will come with a built in explicit justification which people will either accept or not and these decisions will have to be recorded and defended in public most likely with resultant pressures for affected parties to appeal against decisions they do not like).

Comments on the Gillon report

This is also a very valuable report for Pharmac which is broadly in agreement with the thrust of Hansen's argument. It was written with the advantage that the author was able to read and comment briefly on Hansen's approach and conclusions. Gillon, like Hansen, reminds us that social justice lies at the core of the question of how to distribute funding for high cost pharmaceuticals, but that there are many theories of social justice each of which will be supported or criticised by different philosophers and social, religious and other groups. The strength of Gillon's report is the way he sets out in detail a wide range of different approaches to thinking about social justice, each of which has some justification.

He confesses honestly that, 'It would be nice to be able to answer questions about how to deal with conflicting moral values or principles, and how to deal with moral dilemmas, with moral certainty or even with moral confidence, but alas I can't.' Instead, Gillon encourages Pharmac to beware simple solutions to complex judgements. He advocates that Pharmac acknowledge explicitly this wide range of potentially conflicting moral criteria, the lack of a single theory of distributive justice which could reconcile these criteria and to make explicit its own particular approach to resolving such conflicts when they occur.

I have discussed his principal criticism of Hansen's report above, however, he makes one other criticism which I agree with, namely that there is really no difference between Hansen's second and third options ('Equity weighting' and MCDA) for approaching the problem of how to make decisions in the face of multiple, conflicting criteria (pp21-2). Both require some means of identifying and weighting numerically different social justice criteria. Analytically, they seem to me to amount to the same sort of approach.

Gillon recommends that Pharmac makes its distributive criteria and procedures more explicit ex ante, broadly in line with Hope, Reynolds and Griffiths' approach (also supported by Hansen), but that no attempt should be made to use MCDA, not even as a decision aid. Gillon advances to main arguments for this: an empirical argument that MCDA will be rejected by many of those affected by Pharmac's decisions; and a philosophical argument that no single rule cannot be consistently applied to decision making when moral rules are in conflict (Kantian objection). As a result of the latter objection, Gillon regards 'consistency' between decisions as a potential sign of poor decision making rather than a strength since every distributional decision is unique to its particular circumstances. While it is hard, if not impossible, to argue with the Kantian objection, the empirical objection is, in theory, testable in the New Zealand context. It would be possible, for instance, for Pharmac to undertake MCDA, use it as a decision aid to, publish the results of the potential decisions that would result and, in parallel publish the results of potential decisions in which MCDA was not used (i.e. entirely implicit decision processes). The reactions of key stakeholders to the two sets of potential

decisions could then be compared in terms of acceptability, even if consistency per se were not to be regarded as always desirable. Gillon suggests just such an exercise (p14), though without the final step of obtaining the views and reactions of stakeholders.

Gillon recommends the creation of an 'allocation committee' of Pharmac to advise the Board after, and ahead of, potentially contentious decisions. It is unclear what precise role this committee would play. Gillon introduces it as 'advisory', but in describing it, its role seems to expand into being a decision making body using the Hope et al procedures. I was not convinced that the Board of Pharmac would not be better placed in both roles, perhaps with augmented membership in order to ensure that a variety of ethical perspectives are brought to bear on decisions (irrespective of whether MCDA is used to support decisions).

Gillon advocates that Pharmac specify its 'ethical framework'. This is similar to Hansen's plea for Pharmac to clarify its approach (listed on p15). However, Gillon's description of this seems less helpful than Hansen's. I cannot see how what Gillon describes on page 15 of his report would assist with decision making. In fact, he ends up admitting as much in mid-paragraph, saying 'Unfortunately, adoption of neither of these ethical frameworks will resolve the crucial problems .., namely lack of agreement on a substantive theory of distributive justice.' Instead, I would suggest that Pharmac's Board has to decide its own distributive criteria after whatever process of engagement with stakeholders it deems appropriate.

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Moore Report:
PHARMAC decision-making about high-cost pharmaceuticals

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Acknowledgement and disclaimer

I chair the National Ethics Advisory Committee (NEAC), a statutory advisor to the Minister of Health on ethical issues of national significance on health and disability matters. I am also a member of the National Health Committee and the Public Health Advisory Committee; and a member of the Data & Safety Monitoring Board of New Zealand's Health Research Council. The views I express in this report are not necessarily those of any of these organisations. Nor do I have any interest in the success or otherwise of any pharmaceutical company.

1. Executive Summary

Any approach to deciding which pharmaceuticals to fund depends on ethical beliefs. This includes PHARMAC's current approach.

Economic tools such as CUA, and tools of multi-criteria decision analysis such as "Points Wizard", can express with precision the content and implications of a wide range of potential ethical bases for pharmaceutical decision-making.

High cost pharmaceuticals should be defined solely in terms of cost per QALY only if this factor is all that matters in pharmaceutical funding decisions.

This report has not found any clear and persuasive rationale for maintaining a category of high cost pharmaceuticals (HCPs).

PHARMAC should regard the overall aim of its pharmaceutical funding decision-making as being to secure "best value for money" for New Zealanders. The central question then concerns how best to understand "best value".

One aspect of "best value" in pharmaceutical decision-making is benefit. In particular, there is a benefit requirement on all such funding decisions.

There is merit in PHARMAC's interpretation of "benefit" as "health gain", and in its interpretation of "health gain" as "QALY gain". But both these moves depend on substantial ethical beliefs, even granted the "hard" numerical nature of QALY data.

It is plausible that fairness should play a role in PHARMAC decision-making as to the funding of pharmaceuticals.

Allocation according to need should not play any role in PHARMAC decision-making as to the funding of pharmaceuticals, because it is inconsistent with the benefit requirement. Need-weighted benefit, on the other hand, is consistent with the benefit requirement, and is worthy of further consideration.

Allocation according to the rule of rescue should not play any role in PHARMAC decision-making as to the funding of pharmaceuticals, because that rule is inapplicable to PHARMAC's situation.

PHARMAC should give consideration to procedural values in its pharmaceutical decision-making processes, including the following: open and transparent, inclusive, reasonable, responsive, and accountable.

I favour the "four step method" proposed in the Hansen Report, with the qualification that the most general statement of this method should not commit to the QALY measure of benefit.

2. Introduction

PHARMAC is currently considering its decision-making regarding the funding of high cost pharmaceuticals (HCPs). I agreed to provide a contractor report to assist it in this process. This is to be a report:

> containing a commentary on one or both of the Gillon and Hansen reports regarding the funding of High Cost Pharmaceuticals and answering questions posed by PHARMAC.

I also agreed to consider some or all of the following points in my commentary:

a) critique one or both of the Hansen and Gillon reports and act as peer review; and/or
b) build on and extend the reports with a view to how PHARMAC may progress this review; and/or
c) elaborate on the reports in any other way you consider appropriate.

In the main, my report takes approach (b). It is structured around the eight questions that PHARMAC asked Hansen and Gillon to address, and is informed by their contractor reports, and by the background material that PHARMAC provided to them. It also draws on my training in philosophical ethics, and my experience as a member of various New Zealand public bodies concerned with ethics and/or policy advisory matters concerning health and disability services and/or research.

PHARMAC's questions to Hansen and to Gillon were:

(a) What are the main economic/social justice/ethical theories relevant to how decisions on funding "high cost" pharmaceuticals could be made?
(b) What, if any, justification is there for assessing High Cost Pharmaceuticals differently from other pharmaceuticals considered for public subsidisation?
(c) What might be the downsides of valuing High Cost Pharmaceuticals differently?

(d) Could cost-utility analysis be used more effectively when considering "high cost" pharmaceuticals (and others too)?
(e) What, if any, changes do you recommend PHARMAC make to its current decision-making process for "high cost" pharmaceuticals (and others too)?
(f) What role should "rule of rescue" play in assessing High Cost Pharmaceuticals for funding?
(g) What are the arguments for and against paying a higher price (per QALY gained, for example) for pharmaceuticals for those who are worse off clinically with poor quality-adjusted life expectancy, but of arguably greater need (for example, the terminally ill)?
(h) Are there any general comments that you wish to make?

PHARMAC's central question is (e), about possible changes to its decision-making process for HCPs. My report approaches that question in several steps. Section 2 outlines the broad context of HCP decision-making. Responding to question (a), Section 3 then discusses theories relevant to HCP decision-making. Responding to questions (b) and (c), Section 4 discusses possible justifications and downsides of assessing HCPs differently from other pharmaceuticals. Responding to questions (d), (f), and (g), Section 5 considers possible principles for determining the *content* or substance of HCP decision-making. Responding to question (h), Section 6 considers possible procedural or *process* values in HCP decision-making. Each of Sections 3 – 6 includes material on PHARMAC's current approach, on the Hansen Report and Gillon Report, and on potential changes to PHARMAC's HCP decision-making.

3. Current context of PHARMAC decision-making

Pharmaceuticals are proposed to PHARMAC for public funding, but there is insufficient resource for its immediate answer to be "yes" in every case. To a significant extent, this "fact of resource constraint" is driven by rapid development of new medicines, and by continuous identification of new settings for use of existing medicines.

Given the fact of resource constraint, PHARMAC decides which medicines it will fund and which of them it will not presently fund. The decision-making process

extends only to "those in need" (PHARMAC 2001: 6), thus giving "need" a fundamental role. For example, medicines for those who are not in need are not considered, even if they would provide significant "enhancement" benefits.

Amongst pharmaceuticals, PHARMAC distinguishes "high-cost pharmaceuticals" (HCPs) from others. Its current definition is that HCPs are those with "cost per quality-adjusted life year (QALY) … significantly higher than the less than $10,000/QALY of most Pharmaceutical Schedule listings." (PHARMAC 2003: 1-2) "QALYs" or "quality-adjusted life years" are the numbers of years a person lives, adjusted for the quality of those years. Many pharmaceuticals can be expected to *add* QALYs for those whom they treat. The number of QALYs added, and the cost of the pharmaceuticals required to achieve this, varies from medicine to medicine, and across different clinical settings. The lower the cost per QALY, the greater the total number of QALYs that would be gained for New Zealanders from a given budget.

4. Theories relevant to pharmaceutical (including HCP) decision-making

Cost-utility analysis (CUA) currently plays an important role in PHARMAC's decision-making about the funding of pharmaceuticals, including its decisions about HCPs. In particular, its use of CUA enables it to analyse pharmaceutical cost per unit of "utility", and to include this as one important factor in its funding decisions.

To use CUA at all, one must make ethical assumptions. Specifically, one must assume that the more "utility" a pharmaceutical can provide to people the better it is, other things being equal. This assumption also implies that the lower a pharmaceutical's cost per unit of utility the better it is, other things being equal. This is because the lower the cost, the greater is the utility gain achievable within a given budget.

The ethical assumptions one must make to use CUA at all are not controversial. As John Rawls has noted:

> acting from the best reasons, or from the balance of reasons as defined by a moral conception, is not, in general, to maximise anything…. Neither for that matter does the economists' utility function specify anything to be maximised… from a purely

> formal point of view, there is nothing to prevent an agent who is a pluralistic intuitionist from having a utility function. (Rawls 1996: 332, n.42.)

Rawls's point about the ethical neutrality of "best reasons" and of "utility functions" generalises also to "best value" and to "utility", as invoked in any bare decision to use CUA. Use of this tool, in the context of HCP decision-making, does not in itself commit one to any particular moral or ethical conception.

In the above quotation, Rawls points out that even a pluralistic intuitionist view can be expressed in terms of a utility function. According to such a view, there is a plurality of factors that matter for decision-making, and their number and nature needs to be worked out by reflection on our ethical beliefs or "intuitions". In the present context, for example, one pluralistic view is that health gain, extent of need, and rule of rescue are three distinct factors that should all be considered in HCP decision-making. This and other pluralistic views are discussed by Hansen and by Gillon, and also in Section 6, below.

As Rawls notes, pluralistic views can in principle be expressed *within* the notion of "utility", and thus also within use of the CUA tool. Given that this is possible, Hansen's contrast between economics-based approaches and need-based approaches (Hansen 2005: 4) is misleading. His claim that his focus is just on consequentialist views (Hansen 2005: 3) is similarly misleading. Hansen in fact does appreciate these points, because he goes on to show how decision-analytic tools such as "Points Wizard" can represent and operationalise a whole range of normative views, importantly including pluralistic or multi-factor views in which not all factors or criteria are outcome focussed (Hansen 2005: 21-22).

PHARMAC acknowledges that the basis of its HCP decision-making is currently sometimes pluralistic: "Decisions can, and have, been made to treat the needy on grounds other than maximising health." (PHARMAC 2004: 7). On the other had, it also writes that:

> CUA does clarify the size of the efficiency trade-off if a decision to treat the needy is made (where the needy will gain less benefit per dollar spent than patients who

> would benefit from an alternative proposals). However, this is the only exception where CUA informs ethical debate. (PHARMAC 2004: 7)

This indicates that PHARMAC currently interprets "utility" only in terms of beneficial outcomes, and interprets beneficial outcomes only in terms of extent of health gain. Thus: "CUA is a tool for maximising health" (PHARMAC 2004: 7). As noted above, PHARMAC also goes further still, interpreting maximisation of health gain in terms of: "maximising the number of QALYs" (PHARMAC 2004: 10). In short, PHARMAC's current use of CUA is *not* pluralistic. Its *overall* decision-making is therefore pluralistic only if it also considers factors other than the results of CUA analysis. How often does it do that, and how much weight does it then give to those other factors? The fact that it currently defines the HCP category solely in terms of cost per QALY gained suggests that factors other than QALY gain generally do not have much weight in its decisions. More empirical detail on this could perhaps be gained by audit of previous decisions, based on the question of how many medicines with a cost per QALY of greater than $10,000 have been included in PHARMAC's Pharmaceutical Schedules, excluding cases where political request has played a role.

It is a plausible ethical belief that maximisation of health gain does matter and should be counted in HCP decision-making. More controversial is the belief that health gain is best measured in terms of QALYs gained. Also controversial is the ethical belief that health gain is the *only* beneficial outcome that matters and that should be counted in HCP decision-making. There is brief further discussion of these issues below, in Section 6.

Imagine if CUA, with "utility" interpreted solely in terms of QALYs gained, were the *only* factor in PHARMAC's HCP decision-making. Hansen (2005: 3, 10) believes this would equate with a commitment to utilitarianism. But this is a mistake. Standard QALY measures in fact assess only *health* gain, and also assess only gain of this sort for those directly treated. This is a lot narrower than utilitarianism, which considers gains and loses to *all* aspects of well-being or happiness, not just health aspects; and which also considers gains and loses for *all* who are affected, not just for those who are directly acted upon.

Any actual use of a QALY measure depends on a number of substantial ethical assumptions, such as those just noted. Some others are briefly described in the Appendix to this paper.

Which theories or ethical beliefs should we adopt to underpin pharmaceutical decision-making? Hansen surveys some of the main candidates (Hansen 2005: 17-19), but does not make any firm recommendations beyond inclusion of a focus on maximisation of health gain.

Gillon (2005: 15) recommends adoption of an overarching ethical framework of four principles: benefit, non-harm, respect for autonomy, and justice. I am not sure that adoption of any such framework would be helpful. For instance, significant further work would be needed to determine whether these four principles that were tailored mainly for application to doctor-patient decision-making are apt also for the present context of policy for prioritisation decisions amongst substantial groups of people.

Gillon (2005: 1-3) also surveys various ethical theories. Here it is helpful to apply the distinction made by Kagan (1998) between "ethical factors" and "ethical foundations" for those factors. We do need to decide which ethical factors – amongst benefit, need, fairness, and so forth – should underpin prioritisation decisions about pharmaceutical funding. But we need not resolve any of the foundational issues that Gillon considers about Kantian theories, Marxist theories, religious theories, libertarian theories, and the like.

I return below to the question of which ethical factors should underpin prioritisation decisions about public funding of pharmaceuticals. Section 6 considers ethical factors in decision content. Section 7 considers ethical factors in decision process.

Conclusions:

Any approach to deciding which pharmaceuticals to fund depends on ethical beliefs. This includes PHARMAC's current approach.

Economic tools such as CUA, and tools of multi-criteria decision analysis such as "Points Wizard", can express with precision the content and implications of a wide range of potential ethical bases for pharmaceutical decision-making.

5. Different assessment for HCPs?

PHARMAC currently defines High Cost Pharmaceuticals (HCPs) as those with a "cost per quality-adjusted life year (QALY) … significantly higher than the less than $10,000/QALY of most Pharmaceutical Schedule listings." (PHARMAC 2003: 1-2) This definition also suggests a rationale for the distinction – to mark out those pharmaceuticals that are generally not funded, given use of standard criteria.

Can we justify assessment of HCPs that differs from assessment of other pharmaceuticals? Hansen's response is brisk: the same considerations apply to funding decisions about all pharmaceuticals (Hansen 2005: 2). Gillon (2005: 9) instead puts a need-based argument in favour of difference of assessment. A difficulty for his argument, however, is that if need matters, it surely matters to the funding of all pharmaceuticals. But if so, then appeal to need cannot be a basis for distinguishing HCPs from other pharmaceuticals.

A more general idea is that an HCP category might be needed to enable unforeseeable factors to be considered for medicines that would not otherwise be funded. Again, however, if there are such factors then they might arise for medicines in general, and they might in some cases count *against* medicines that would otherwise be funded. A better way of handling such possibilities would be to make decision-making processes for all medicines sufficiently flexible to address any such factors that might arise.

A different idea is that it is unfair to patients to make their level of access to medicines depend on things they do not control, and they typically do not control the cost of the medicines they need. A difficulty for this line of thought, however, is that patients also often have little control over the extent of benefit, the extent of their need, or the extent to which theirs is a "rescue" situation. I conclude that no ground has yet been given for *special* consideration for level of cost.

A related idea is that it is unfair to patients to leave them with no hope of publicly funded support; and if HCPs are assessed in the same way as other medicines, then patients who need them are indeed left with no hope of publicly funded support. There are difficulties with this argument too. Creation of an HCP category does not alter the fact of constrained resources, and given that fact, any explicit decision process is bound to leave some patients explicitly without publicly funded medicines. If we assume that HCPs are defined as having relatively low value for money (however "value" is best to be interpreted), and if we assume also that there is a given overall pharmaceutical budget, then creation of an HCP category will in fact tend to *increase* the overall numbers of patients who are left without hope of publicly funded support. This suggests that introduction of such a category actually tends to exacerbate the problem of hopelessness.

Consider this further argument in favour of establishing and retaining a category of HCP. It is desirable to maximise the extent to which pharmaceutical funding decisions are taken *within* PHARMAC's decision-making arrangements. Retaining an HCP category can be expected to help minimise the number of cases that are effectively decided outside these arrangements - for example, by being decided through policy-maker funding directives to PHARMAC. This argument merits further consideration, but it is not easy to evaluate. It seems to invoke possible effects on the stability and integrity of overall decision-making. It is not clear, however, how an HCP category can be used to secure the suggested effect.

As is suggested above, it is best to define any HCP category in terms of relatively low value for money. This is a matter of high cost per QALY only if QALY gain is confirmed to be all that matters in pharmaceutical funding decisions.

If there is to be any HCP category, its decision criteria will either be the same as, or will differ from, those for pharmaceuticals in general. If the former, then the introduction of the HCP category will tend to generate unfairness for those at the bottom end of the general category, by making them tend to miss out despite scoring better than even the top candidates in the HCP category. If the HCP decision factors or criteria differ from those for pharmaceuticals in general, on the other hand, then a rationale is needed for the difference of criteria. Some arguments for such a difference

have been canvassed above, but none of them clearly establishes its case. In short, I have not found any clear and persuasive rationale for retention of any HCP category. For this reason, discussion below generally proceeds in terms of pharmaceutical decision-making, rather than in terms of HCP decision-making in particular.

Conclusions:

High cost pharmaceuticals should be defined solely in terms of cost per QALY only if this factor is all that matters in pharmaceutical funding decisions.

This report has not found any clear and persuasive rationale for maintaining a category of high cost pharmaceuticals (HCPs).

6. Content of pharmaceutical (including HCP) decision-making

Hansen argues (2005: 1, 3) that: "All approaches to deciding which pharmaceuticals to fund, including high cost ones, are inherently *normative* in nature", and that we need to examine which values such decisions should depend upon. I agree. This section briefly comments on some of the main candidate values, normative considerations, or ethical factors - taking these three terms to be near synonyms.

PHARMAC's current decision criteria are: the health needs of all eligible people within New Zealand; the particular health needs of Maori and Pacific peoples; the availability and suitability of existing medicines … and related things; the cost-effectiveness of meeting health needs by funding pharmaceuticals rather than using other publicly funded health and disability support services; the budgetary impact of any changes to the Schedule on the pharmaceuticals budget and the Government's overall health budget; the direct cost to health service users; the Government's priorities for health funding; and such other criteria as PHARMAC thinks fit (subject to a consultation requirement). (PHARMAC 2001: 4-5)

I agree with Hansen (2005: 15) that, as stated, these criteria are too high-level to be implemented in decision-making. I also agree with Hansen that a helpful approach is to see the overall aim as being to secure "best value for money" from pharmaceutical

funding decisions. The operational account of "value" can then be worked out on the basis of the high-level criteria, in terms of factors that might include health gain, health need, and rule of rescue. On such further detail, see below.

Note also that the high-level PHARMAC criteria are directed at the content or substance of funding decisions. With the exception of the consultation requirement in the last criterion, they are not directed at the process or procedure of pharmaceutical decision-making. These matters are discussed further in Section 7, below.

The rest of this section comments on candidate factors in the substance of pharmaceutical decision-making.

5.1 Benefit

Benefit is an important factor in pharmaceutical funding decisions. PHARMAC currently interprets this aim rather narrowly, in terms of: "Best health outcomes for those in need" (PHARMAC 2001: 6). Counting only health gain conflicts with the utilitarian claim that *all* benefit to individuals matters. For example, it excludes gain of income, such as the difference a medicine might make to the time taken for those in paid employment to get back to work. It also discounts the fact that a medicine that gives a happy person an extra healthy year will actually be more beneficial than a medicine that gives an unhappy person an extra healthy year.

But it is arguably a good thing that PHARMAC has a narrow account of benefit. In particular, it might be thought unfair to count aspects of benefit other than health gain. But if so, this is implicit recognition that fairness – or something similar – should *also* be a significant factor in the substance of pharmaceutical decisions, even if only in the role of limiting the kinds of benefit that should be allowed to count.

As noted earlier in this paper, PHARMAC has also adopted a QALY measure of health gain. QALYs measure this in terms of the extra years of life that a course of action would give people, adjusting these extra years for quality so that better years count for more than worse years.

There is some evidence that QALY tools for assessing health gain are not always sensitive to patient-experienced health status or to improvements in health status. (Derrett, Paul, et al 2002; Derrett, Devlin, et al 2003) On the other hand, PHARMAC must make prioritisation decisions across different conditions and diseases, different medicines, and patients' health experience over widely divergent clinical settings. Condition-specific measures of health gain cannot be used to make such wide-ranging prioritisation decisions, even if they might at present be more sensitive than QALY measures to important aspects of health gain. Still, if a credible condition-specific measure, drawing on patient experience, were to assess the health gain from one medicine as greater than the health gain for another, and yet a QALY measure were to generate the opposite finding, this would raise doubt about the reliability of the QALY measure. This matter cannot be fully investigated here, but it should raise a caution about the uncritical use of QALY measures. For example, where there are only small differences in the assessed QALY gain per dollar of two or more medicines, these differences should probably not be thought reliable indicators of genuine difference of health gain. More generally, one should not be misled by the "hard" numerical character of QALY data into supposed that these numbers are highly robust.

A further worry sometimes expressed about QALY measures concerns their handling of disability. In generating QALY values for health states, it is common practice to appeal to the judgments or preferences of relevant groups. But which groups? Might not those with little or no experience of disability tend to be prejudicially averse to states of disability? (Murray 1996) On the other hand, might those who have disabilities be so well accommodated to their situation that they give inappropriately deflated estimates of the benefit of medicines in this area? The issues cannot be pursued further here. Again, they generate grounds for caution about the robustness of QALY-gain data, and grounds for ongoing liveliness to the possibility that rival measures of benefit might at some point supplant QALY measures.

Let me conclude the sub-section with a larger point. There can be little doubt that benefit should be an important factor in pharmaceutical decision-making. As Raanan Gillon (2005: 4) observes in his generally sceptical assessment of the role of benefit: "There is no point in, and in the context of scarce resources no moral justification for,

providing treatments that cannot provide benefit." This states a *benefit requirement* on all such prioritisation decisions. I endorse Gillon's claim that there is such a requirement.

5.2 Fairness

QALYs are only concerned with benefit. They do not take account of fairness. As was noted in the previous sub-section, however, it is natural - in fairness - to limit the kinds of benefit that can count in pharmaceutical funding decisions. There might also be other fairness considerations, going beyond maximisation of health gain. In short, funding treatment of those whose health would benefit most is not necessarily the fairest thing to do, and it is plausible that fairness matters.

Arguably, one aspect of fairness is the reduction of inequalities. The view that prioritisation decisions should contribute to reducing inequalities has been persistently expressed in the New Zealand debate on prioritisation, including in recent documents (eg., Ministry of Health 2005). Such goals are perhaps implicit also in PHARMAC's operational expectation that it attend particularly to Maori and Pacific health. If "inequalities reduction" is the rationale for these expectations, however, PHARMAC should operationalise this ethical factor in a more general form. Maori and Pacifica communities do indeed face significant health inequalities. Other identifiable communities do so too, including refugee and migrant communities, and disability communities.

Note that inclusion of inequality reduction as a factor in pharmaceutical decision-making would influence a particular decision only if evidence were provided that the medicine in question would provide more value to identifiable groups that face health inequalities than it would provide on average to the population in general.

5.3 Need

It was noted above that need already plays a fundamental role in PHARMAC's current decision-making: only those who are in need are even to be considered for

publicly funded pharmaceuticals. Hansen (2005) notes, and Gillon (2005) more fully discusses, several further potential roles for need. I comment briefly on these below.

Gillon considers the idea of allocation in proportion to need (Gillon 2005: 5, 7). As stated, this idea is unconstrained by whether or not we can actually do anything to meet any of the need in question. The trouble is that this in consistent with Gillon's own benefit requirement. Recall (Gillon 2005: 4): "There is no point in, and in the context of scarce resources no moral justification for, providing treatments that cannot provide benefit."

An obvious alternative to the rather extreme idea of allocation in proportion to need, irrespective of our capability to meet it, is the idea of need-weighted benefit. In support of this alternative view, Gillon (2005: 9) argues that if one rejects it, one is committed to the view that, benefit for benefit:

> It doesn't matter whether the need being met by the pharmaceutical is a minor one (the itching and soreness between two toes of a minor case of athlete's foot, for example) or a major one (the pain of a heart attack for example).

The idea of need-weighted-benefit is consistent with the benefit requirement, and it is worthy of further consideration, including consideration of whether there are any robust measures of health need available for operational use. In principle, it would not be a stretch to think of "need-weighting" of benefit as being an aspect of fairness.

5.4 Rule of Rescue

Roughly speaking, the rule of rescue states that we have special ethical obligations to aid those who are here and now rather than elsewhere and in the future, or who are known to us rather than of unknown identity, or whom we can directly aid by immediate action rather than through indirect and eventual aid. For further discussion, see, for example, Kagan (1998: 133-137).

In my view, rule of rescue cannot appropriately be applied to PHARMAC decision-making about the public funding of pharmaceuticals. The reason is that the conditions

for application of the rule are not satisfied in this case. PHARMAC is always faced here and now by greater numbers of people who could in principle be aided by publicly funded pharmaceuticals than it can in practice fund from its budget. This is a general fact of the matter, not limited to the current level at which that budget happens to be set. In general, these same people are also similarly placed with respect to one another on the dimensions of known versus unknown, and accessibility to direct aid. Furthermore, even if it is ethically defensible for individual citizens to express the partiality that is central to the rule of rescue, public bodies must treat citizens in an even-handed and impartial manner. This rules out use of the rule of rescue as any part of the basis of decisions as to public funding of pharmaceuticals.

Conclusions:

PHARMAC should regard the overall aim of its pharmaceutical funding decision-making as being to secure "best value for money" for New Zealanders. The central question then concerns how best to understand "best value".

One aspect of "best value" in pharmaceutical decision-making is benefit. In particular, there is a benefit requirement on all such funding decisions.

There is merit in PHARMAC's interpretation of "benefit" as "health gain", and in its interpretation of "health gain" as "QALY gain". But both these moves depend on substantial ethical beliefs, even granted the "hard" numerical nature of QALY data.

It is plausible that fairness should play a role in PHARMAC decision-making as to the funding of pharmaceuticals.

Allocation according to need should not play any role in PHARMAC decision-making as to the funding of pharmaceuticals, because it is inconsistent with the benefit requirement. Need-weighted benefit, on the other hand, is consistent with the benefit requirement, and is worthy of further consideration.

Allocation according to the rule of rescue should not play any role in PHARMAC decision-making as to the funding of pharmaceuticals, because that rule is inapplicable to PHARMAC's situation.

6. Process of pharmaceutical (including HCP) decision-making

The previous section considered candidate ethical factors to inform the content of PHARMAC decisions on funding of pharmaceuticals. This section will more briefly consider candidate ethical factors to inform the process of PHARMAC decision-making.

PHARMAC (2004: 7) comments that: "CUA results are considered a guide to decision making, not a substitute". In general, however, PHARMAC does not provide much commentary on this process side of its decision-making. Similarly, except for his discussion of a proposed "four step method" – considered briefly below - Hansen is concerned only with distributional justice, and leaves aside issues of procedural justice (Hansen 2005: 3). Gillon does consider some process issues, but only briefly, without considering the values or ethical factors that might underpin these matters.

In their influential work on "accountability of reasonableness", Daniels and Sabin (1998) aim to design conditions to ensure that resource allocation decisions are made according to rules or reasons that fair-minded people can agree are relevant. In short summary, they favoured: process agreed by all stakeholders, relevance to diverse needs of a defined population, decisions made and the reasons for them are publicly accessible, decisions are based on appropriate evidence and information, and there is provision for challenge and dispute resolution. This work has also recently been built upon in the New Zealand setting of prioritisation decision-making (eg., National Health Committee 2005).

In the rather different setting of pandemic planning, two Canadian documents have identified the following process values to inform decision-making. See University of Toronto Pandemic Influenza Planning Group (2005: B2); and Ontario Health Plan for an Influenza Pandemic (2005: 9):

> Reasonable: Decisions should be based on reasons (i.e., evidence, principles, and values) that stakeholders can agree are relevant to meeting health needs…. The decisions should be made by people who are credible and accountable.
>
> Open and transparent: The process by which decisions are made must be open to scrutiny, and the basis upon which decisions are made should be publicly accessible.
>
> Inclusive: Decisions should be made explicitly with stakeholder views in mind, and there should be opportunities to engage stakeholders in the decision-making process.
>
> Responsive: There should be opportunities to revisit and revise decisions as new information emerges…. There should be mechanisms to address disputes and complaints.
>
> Accountable: There should be mechanisms in place to ensure that decision makers are answerable for their actions and inactions. Defence of actions should be grounded in the other ethical values proposed above [including "substantive" values not quoted here]

The above procedural values seem apt for application also to PHARMAC decision-making for public funding of pharmaceuticals. On the face of it, they are applicable to the process of designing the pharmaceutical funding decision-making process itself, as well as to the process of making decisions within it.

Finally, I comment briefly on the "four step method" originally proposed by Hope, Reynolds & Griffiths (2002), and adapted by Hansen as a proposed approach to decision-making for the funding of pharmaceuticals (Hansen 2005: 16-17). In general terms, I support this method, including the refinements proposed by Hansen. It would be better, however, if "cost per QALY" and related expressions were replaced throughout by "cost per unit of value". This would leave open, and perhaps to be settled by PHARMAC's current review, the matter of how best to interpret "best value for money". In this most general statement of the four step method, QALY

interpretations of its content should join all the other contenders that are arguing for their place in the sun.

Conclusions:

PHARMAC should give consideration to process factors or procedural values in its pharmaceutical decision-making processes. These might include making decisions through processes that are: open and transparent, inclusive, reasonable, responsive, and accountable.

I favour the "four step method" proposed in the Hansen Report, with the qualification that the most general statement of this method should not commit to the QALY measure of benefit.

Appendix: Ethical assumptions in QALY measures

Built into standard QALY measures is the assumption that a given health gain is not any better for people at one time of life than at another. But might not good health enable one to live better at one time of life than at another - eg., when one is having and rearing a young family?

Another standard assumption in QALY measures is that benefit is independent of length of life. But might not some things be better if life is going to be short - eg., physical mobility - and others - eg., freedom from pain - be better if life is going to be long? (See Broome 1999)

There are two ways to produce more QALYs – prolong life or make it go better; or bring it about that more QALY-generating lives are lived. Medicines often do both these things. For example, if life-saving medicine is funded for children, then on average those children saved will each eventually have about two children of their own, who will in turn on average have 75 or 80 years of reasonably healthy life, perhaps including having children of their own; and so on. Understandably, the usual approach is not to count any such people or their QALYs at all. This would imply, for example, not counting any of the additional QALYs generated by the additional healthy live births that a better fertility medicine would produce. On the other hand, standard methods of QALY assessment give high value to medicines for the very young – eg., to medicines that save babies who are born prematurely. Crucial to this is the matter of whether a medicine treats an already existing person, or instead helps produce one who didn't previously exist. Though QALY measures treat these two cases very differently, it is of course a matter of major ethical controversy where the line falls between the two sorts of case. For instance, on which side of the line should we put a medicine that reduces miscarriage rates? Note also, however, that all measures of benefit, including all measures of health gain, face this difficult ethical issue. It is by no means unique to QALY measures.

One apparently technical assumption, often made in the design of QALY measures, is that the "quality adjustment" weighting of life years gained cannot fall below zero. This excludes *a priori* the possibility that in prolonging a patient's life, a medicine

might in a small range of cases actually make life go *worse* for the patient. It is ethically controversial that quality of life can be so bad that it makes life go worse for one; but it is also ethically controversial that all patients who state this view about their own situation must be mistaken.

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ETHICS AND HIGH COST PHARMACEUTICALS

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Introduction

This is a critical review primarily of Raanan Gillon's report, although it makes some reference to Paul Hansen's. This review covers three topics: the list of theories of justice (pp. 1-3 in Gillon's report); Gillon's views on high cost pharmaceuticals (HCPs) (pp. 9-14 in the report); and his recommendations to Pharmac (pp. 14-16 in the report).

In the comments on the lists of theories, I ask why Pharmac wanted this; comment on the content of the lists; point out what a mess things seem to be; claim that things are in some ways even worse than would appear from these reports; and then find some more constructive things to say.

In discussing Gillon's views on HCPs, I expound his arguments and offer some criticism. I also point out a gap in his discussion, illustrated by the problem of `orphan diseases'. I then comment on the relation of this problem to the `rule of rescue'.

The discussion of his recommendations will be both brief and enthusiastic.

The list of theories

Pharmac wanted to know `what are the main economic/social justice/ethical theories relevant to how decisions on funding `high cost' pharmaceuticals could be made?' Gillon responded with a long list of options, with anything from a short paragraph to a clause of description. He said that these theories can all be taken to endorse a formal principle of equality, but that the way in which they elaborate this principle makes them inconsistent with each other. He then, quite sensibly in my view, largely ignores this list and considers, in the context of HCPs, only a principle of meeting needs, some conflicting considerations, rescue, and consequentialism. (I shall assume that any reader of this has already read the main reports, so I shall not define these terms.)

It is reasonable of Gillon to have provided the list and the mini-descriptions, given Pharmac's request. But then one has to ask: what was the point of the request? I suppose it would be convenient to have a kind of glossary of terms. My worry is that a certain incorrect picture of moral reasoning is presupposed by the request, namely, that the way to get the right answers in a problem in applied ethics, like the funding of HCPs, is to take some high level theory, plug in the facts, crank the handle, and get an answer. This is incorrect because even a sophisticated grasp of a high level theory plus some facts is not enough to produce a reliable answer. Perhaps we should be less confident of the high level theory than our intuitions in particular cases. After all, as moral philosophers frequently note, we can be much more confident *that* slavery is wrong than we can about any underlying theory (autonomy, utilitarianism, or whatever) that purports to explain this judgement. A second point is relevant: different theories might have the same answer in a certain case, as they do with slavery, even as they disagree about why. Since all of these theories have their believers and, indeed, have something going for them, it is

usually more profitable to avoid the high level disagreement and try to resolve more directly relevant disagreements.

Even if applied ethics should be done by taking a theory and plugging in the facts, it could not possibly be done by people who have just read the list because even if they think they understand the theories, they would not. An analogy is trying to build an aeroplane on the basis of some facts about materials and a paragraph on Newtonian physics.

So much for the advisability of Pharmac's question. What should we make of the list? The impression it must give to non-philosophers (and maybe philosophers too) who want an answer to the questions raised by HCPs is: what a mess! Look at all these different and conflicting views! If we cannot agree on any, how can we decide about HCPs? In fact, things are both worse and better than they appear. I begin with the gloomy side.

First, the list is not complete in that there is yet another plausible view of ethical decision-making that you ought to know about. The basic thought behind it is this: we are all likely to be ill and certainly will die at some point. We all care about our health. But we also care about other things. Resources being scarce, we have to choose how to allocate them across our lives. The problem of just health care can be thought of not as a problem of how much the healthy owe the sick, but how much insurance, or quasi-insurance, we would each like against sickness. You and I will not have the same preferences, so our decisions would not be the same. We may though agree on some things – to take Gillon's example (p.11), we probably would not want any money set aside for cancer treatment for us if we were in a PVS if it meant giving up resources now.

On this type of view, there is no right answer to a question about spending on HCPs independent of what people's actual preferences are.[1] Clearly, much more needs to be said and I do not think this model is without serious problems. However, it is another major contender, so it is another thing to bear in mind.

The second problem is that access to pharmaceuticals is only one aspect of just health care which is, in turn, only one aspect of a theory of justice. Take, for instance, a view which says that priority should be given to the worst off (a `priority view').[2] Suppose I have worse access to pharmaceuticals than you but better access to hip operations. A priority view applied just to pharmaceuticals would give me the priority. But why just consider pharmaceuticals on their own and not the whole package of health care? And why then consider health care alone: what if you have better access to health care but worse access to education than me? The problem here can be put as a dilemma: either a theory of justice is applied by Pharmac only within its sphere of competence, in which case it might make people's overall shares more unjust not less; or else there has to be some overall approach to justice, which would face formidable institutional obstacles (who would do it and how?) and conceptual ones (how do we compare access to hip operations with access to primary schools?).

Certain theories of justice might avoid the dilemma. Utilitarianism does: it counts gains in welfare as gains not matter to whom they occur. If money on a pharmaceutical promotes my welfare more than yours even though I am already better off in other

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[1] Some of the writers who elaborate this `insurance' model (it does not have any official name that I know of) are: Thomas Schelling `The Life You Save May be Your Own' in his *Choice and Consequence* (Harvard, 1984); Allan Gibbard `Health Care Needs and the Prospective Pareto Principle' *Ethics* 94 (1984); and Ronald Dworkin `Justice and the High Cost of Health' in his *Sovereign Virtue* (Harvard, 2000).

[2] As described in Derek Parfit's very important article, `Equality or Priority?', reprinted in M. Clayton and A. Williams (eds.) *The Ideal of Equality* (Palgrave, 2002).

respects, then according to utilitarianism, I should get money spent on me. But in saying this, utilitarianism is often thought to be unjust in ignoring the existing distribution, as Gillon and Hansen point out.[3]

There are other big problems at a theoretical level that make it hard to produce an ethical theory for the allocation of resources to pharmaceuticals. But things are also somewhat better than one might think from the long list Gillon provides. In the first place, the list can be cut. Some of the theories of justice, such as the anti-racism and the environmental theories, can be dropped on the double basis that they are not fundamental theories in their own right, but applications of some higher level theory, and that they are unlikely to have anything to say about HCPs. The list can be further cut when we realize that some of the theories are about the content of justice e.g. utilitarianism, strict egalitarianism, while others are about the source of justice, e.g. religious theories. So, for instance, utilitarianism might be supported by a religious theory or a secular one, or neither. A theory about content does not compete with a theory about source and putting theories of both types as separate entities in the list makes it appear that there is more disagreement about what justice requires than there is.

Another cheering point is one that I made earlier: often there can be agreement on some practical policy even when there is disagreement about its justification. So we may not need to decide between rival theories to accept that some policy is ethically justified.

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[3] Michael Walzer's view, as set out in his *Spheres of Justice* (Martin Robertson, 1983) might also be thought to provide a way out of the dilemma. Walzer thinks that different goods, like health, money, jobs, and love, come with different principles of justice. He thinks justice has no reckoning of overall shares, so we need not compare access to health care with access to education. However, in addition to the serious problems with his account, it is not plausibly applied to a pharmaceutical budget within a health budget. There is nothing sufficiently distinctive about pharmaceuticals that warrants a separate principle of justice.

I also have a constructive proposal. It is reasonable to say that Pharmac takes consequentialist considerations as its default position. My proposal is that Pharmac indeed ought to take consequentialist considerations as its default, and focus only on the problems with consequentialism that are directly relevant. Every moral theory gives some role to consequentialist considerations; all bar consequentialism consider other factors too.[4][5] But not all of these other factors are relevant to HCPs.

Consider some factors that Gillon mentions. He cites Bernard Williams's case of Jim and Pedro, which could be interpreted either as making the point that we should be more concerned about what we do than let happen and\or that it is better to let other people violate negative rights than it would be for us to violate negative rights ourselves.[6] The apparent consequentialist view that negative rights may be violated for more good might be thought a reason to reject consequentialism.[7] But it is not relevant to Pharmac, who would not be violating anyone's negative rights in either funding or failing to fund an HCP. So Pharmac can ignore Jim and Pedro in applying consequentialist considerations. Nor are considerations of autonomy any ground for giving up

[4] I use the wordy term `consequentialist considerations' rather than consequentialism to try to emphasize the ecumenical nature of my proposal: one does not have to be a consequentialist to accept it.

[5] At this point I want to grumble about the distinction Hansen endorses and attributes to Harvey (Hansen, p.3). Hansen says that ethical positions are either consequentialist or deontological. This is false, except insofar as `deontological' is to be defined only as `not-consequentialist'. Consequentialist theories come in lots of different forms, as Hansen points out, but they do have something in common: a commitment to maximizing value. Not-consequentialist theories only have in common the negative feature of denying consequentialism. They do not all accept anything like what Hansen calls `deontological', which is some mix of the priority of duty and a role for intention. Virtue theories, for instance, make virtue prior. Rights theories make rights prior.

[6] In fact, the Jim and Pedro case is a peculiar one, because each of the villagers wants Jim to choose to shoot one, and so they might be said to waive their right that Jim not kill them. Contrary to what Gillon says, Williams himself thinks Jim probably should shoot; he just thinks that utilitarianism gets the answer too quickly. See J.J.C. Smart and B. Williams, *Utilitarianism: For and Against* (Cambridge University Press, 1973), p. 117. A better case would be where Jim can shoot one non-consenting person or Pedro will shoot 19 different people.

[7] Although consequentialists often claim they can support rights. See L. W. Sumner *The Moral Foundation of Rights* (Clarendon Press, 1987) ch. 6.

consequentialist considerations in deciding on funding HCPs. Gillon makes two absolutely correct points in his discussion of autonomy and meeting needs. One is that people can autonomously refuse to take pharmaceuticals. This is not in dispute and not relevant to whether to fund a pharmaceutical for which there is demand. The other is that considerations of autonomy do not give people a claim on having their favourite pharmaceuticals funded. Again, autonomy is another factor that Pharmac can largely ignore in applying consequentialist considerations. Nor do other factors that Gillon mentions give much reason for Pharmac not to stick with these considerations.

Gillon considers a principle of meeting needs, rather than consequentialism, as potentially in conflict with autonomy (although meeting needs might be taken as one version of consequentialism). He also considers special relationships, between doctor and patient, and among co-nationals, which are possibly a problem from the point of view of consequentialism. However, the point about co-nationals sets the scope for Pharmac's funding: it is within New Zealand. But within that scope, it is not relevant to how funding should be allocated. And the point about the special relationship of doctors to their patients seems largely irrelevant to Pharmac, which does not have particular patients. This does leave Gillon's interesting point that Pharmac might have a special duty to continue funding over and above what consequentialist considerations would permit. This could be explained by the idea is that Pharmac would then have a special relationship with these patients. There might well be something in this.[8]

Taking consequentialist considerations as the default is obviously no complete answer, not least because consequentialism does come in different forms. A

[8] Gillon also says, on p. 8, that `[e]limination and prevention of morally unacceptable use and distribution of scare resources can also conflict with distribution in proportion to need', but I did not really understand this part.

consequentialist can attach weight to equality, or to giving priority to the worst off, for instance, or simply be a utilitarian (the best known version of consequentialism).[9] Thus consequentialists may or may not want to maximize QALYs. But the advantage of beginning with consequentialist considerations is that it gives a reasonably high level basis for Pharmac to start thinking about distribution and it suggests a method for proceeding, which is to ignore irrelevant opposing considerations and theories. I shall now go into some of the relevant opposing considerations below, but largely leaving aside these points about consequentialist considerations.

Gillon on HCPs

Suppose a pharmaceutical costs more than $10 000\QALY (or whatever level Pharmac decides warrants special scrutiny.) Should we take it that it simply should not be funded? Gillon says we should not. He has three main reasons. An HCP should perhaps be funded when:

(1) It goes to people who are very badly off. His example is palliative care.

(2) It saves lives.

[9] Here I cannot resist complaining again, this time about both Gillon and Hansen's account of utilitarianism as aiming for `the greatest good of the greatest number'. This slogan is incoherent, in containing two maximands. It is like saying `the prize goes to the person who writes the longest essay in the shortest time'. Who gets it? Someone who writes four volumes in 10 years or someone who turns in a paragraph after five minutes? The double maximand says `both', which is incoherent. To have a double maximand is not merely to say that two things are important; it is an error of formulation, which philosophical utilitarians have known about at least since the economist Edgeworth pointed it out 125 years ago. Philosophical utilitarians do not use `the greatest good of the greatest number'. See James Griffin, *Well-Being* (Clarendon, 1986), pp. 151-4.

(3) It is recommended by a rule of rescue (although he is more ambivalent about this).

The points about the badly off and life saving show disagreement with the instruction to *maximize* QALYs, but may be recommended by some different version of consequentialism. The rule of rescue seems different, and I shall return to it.

Should priority be given to the worst off, that is, should their QALYs count for more? It is certainly plausible, and I do not have any quick argument one way or the other. Should priority be given to saving lives? Gillon believes that lives should get some priority, but not absolute priority. I do not think Gillon's own major argument for this is persuasive. He says that a `sophisticated' utilitarian will attach more weight to lifesaving than a QALY measure gives because life is a necessary condition for having any quality. This sophisticated utilitarian simply sounds confused. The utilitarian wants more utility. If one option gives more utility than another, it is better. It does not matter *how* the utility comes about. That said, Gillon's conclusion and sense of struggle are both plausible. The very tricky problem, for priority to the worse off or life saving, is in saying how much priority either should get. As I think is widely recognized, there is no conclusive answer to this problem.

One problem that Gillon does not mention is that of orphan diseases, that is (in the US anyway) a disease suffered by fewer than one in 1500 people.[10] Suppose money could be spent on a drug that would help a few people or on a different drug that would help more. Suppose that it is no worse to suffer the rare condition than the more widespread

[10] My thanks to the Wollongong philosopher David Neil for telling me this, and giving me this example: `Jumping Frenchmen of Maine', an orphan disease where the sufferers have a massively over-developed startle reaction. The disease was originally associated with French Canadian lumberjacks in Quebec and Maine.

one. Suppose too that the effect on either quality of life or life saving would be identical. In that case, QALY-maximization would say: spend the money where it would help more people. But so too would a QALY view that gave priority to the worst off (if all are equally badly off, better to help more rather than fewer) and a view that gave priority to saving lives (if the drug would save lives, better to save more lives rather than fewer). Perhaps the conclusions of these view is correct: when matters are as described here, or even nearly so, do not give money for expensive pharmaceuticals for orphan diseases. On the other hand, perhaps not. It might be thought that there is something in this complaint: why should I suffer more simply because my disease is rare?

This critical review would not be the place to give an answer of my own, even assuming that I had one. A place within moral philosophy to look for one is the discussion of whether, when a choice has to be made among strangers, it is right to save the greater number. To reiterate, what is being looked for are considerations of fairness that are not reducible to priority either for the worst off or to saving lives.[11]

Whatever these considerations of fairness turn out to be, I suspect that they are the rational kernel in the so-called `rule of rescue'. As Gillon makes clear, this `rule' is both a tangle in its content and lacks any clear justification. One interpretation might be that the rule is simply a generalization from people's psychological reactions, rather than a justification of them. People are more concerned about photogenic people and animals than ugly ones: but `To each according to her photogenicity' is not much of a moral rule. On this interpretation, the most, morally, that can be said for the rule of rescue is that we

[11] The famous article that started the modern debate is John Taurek's `Should the Numbers Count?' *Philosophy and Public Affairs* 6 (1977). See also Derek Parfit's reply, `Innumerate Ethics' *Philosophy and Public Affairs* 7 (1978). The major book-length discussion is F.M. Kamm's *Morality, Mortality* vol.1 (Oxford University Press, 1993).

have to take account of people's unjustified reactions because otherwise they will do even worse.

I think there is a lot of confusion and gut reaction in the rule of rescue that simply does not survive careful thought. But there is also the sense, that Gillon has too, that we cannot simply abandon people in front of us who could be helped, even if this would save other people or more people in the future. A familiar variant of the problem is this: should we spend a large sum of money to help some miners who are trapped now, or should we abandon them and spend the same sum on mine safety that we know would save a greater number of miners in the future? In practice, it is hard for people to say that the trapped miners should be abandoned, and, significantly, this reaction is often not regarded as a regrettable irrational error that they wish they could not commit. People might think of their tendency to commit the gambler's fallacy or eat too much ice cream as something they wish they did not do, but they do not think of their reaction to the miners' case in this way. This suggests that there is something in the rule of rescue beyond mere unthinking reaction although, to my mind, there is unthinking reaction as well.[12]

Gillon's recommendations

Gillon makes the following recommendations to Pharmac: that Pharmac have a list of the values it takes into account; that it should not look to these values to provide an algorithm

[12] An accessible and brief account of the difficulties with rescue-type views is in Jonathan Glover's well-known *Causing Death and Saving Lives* (Pelican, 1977), pp. 210-13.

for decision-making and that Pharmac should instead rely on judgement; that a points program (of the type that Hansen recommends) should be used in a research capacity, to see what recommendations it comes up with, but that the recommendations should not be taken to be binding; that Pharmac's judgement should be made with the aid of an allocation committee and\or citizen's juries; that it should have an appeals mechanism; and that it should accept that whatever it does will produce moral dissatisfaction.

On the face of it, these all seem very sensible recommendations. I do not know how they compare with the current structure of Pharmac's decision-making, so I do not think I can unhesitatingly endorse them. But they are based on a sound view of the relation between rules and moral judgement; in particular, that there is no avoiding judgement. They draw support from the point made by both Gillon and Hansen, that the questions of HCPs is fundamentally normative. Pharmac should be reasonably explicit to itself about the values on which it bases its decisions and, given that it uses public money, it should be explicit to the public as well. It might also be that a reasonably transparent framework and such independence as an allocation committee or citizen's jury might have would allow even people who are disappointed by Pharmac's decisions at least to feel that they have been treated fairly.