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

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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

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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).

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\(^3\) 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) 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. 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.

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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 CUA’s “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, 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 $h^0_x$ QALYs in the diagram. Similarly, without Drug Y, Patient Group Y would enjoy just $h^0_y$ 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. $h^0_y < h^0_x$).

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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 $h^\text{Y}_{\text{max}}$ QALYs and Patient Group X would be stuck with $h^\text{X}_0$. 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 $h^\text{X}_{\text{max}}$ QALYs and Patient Group Y with $h^\text{Y}_0$. 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. $h^\text{Y}_0 < h^\text{X}_0$), Patient Group X has a greater capacity to benefit from treatment than Patient Group Y (i.e. $h^\text{X}_{\text{max}} - h^\text{X}_0 > h^\text{Y}_{\text{max}} - h^\text{Y}_0$). (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, $h^\text{Y}_{\text{max}} - h^\text{Y}_0 > h^\text{X}_{\text{max}} - h^\text{X}_0$), 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.

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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 argues 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 maximisation of the minimum (or worst possible) outcomes.

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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.
In the present context, 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).

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 $h^0_Y/h^0_X$ (i.e. their relative health needs). As can be seen in the figure, Patient Group $Y$, which is needier (i.e. $h^0_Y < h^0_X$), 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 ($h^0_Y$ and $h^0_X$). 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

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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).\(^\text{18}\)

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

\(^\text{18}\) 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 (Metcalf 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.

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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).\textsuperscript{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 (Edward 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

\textsuperscript{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). 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, 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

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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

<table>
<thead>
<tr>
<th>Cost per QALY</th>
<th>Points</th>
<th>Score</th>
</tr>
</thead>
<tbody>
<tr>
<td>over $250,000</td>
<td>0</td>
<td></td>
</tr>
<tr>
<td>$200,001 to $250,000</td>
<td>40</td>
<td></td>
</tr>
<tr>
<td>$150,001 to $200,000</td>
<td>59</td>
<td></td>
</tr>
<tr>
<td>$100,001 to $150,000</td>
<td>83</td>
<td></td>
</tr>
<tr>
<td>$50,001 to $100,000</td>
<td>86</td>
<td></td>
</tr>
<tr>
<td>$20,001 to $50,000</td>
<td>67</td>
<td></td>
</tr>
<tr>
<td>$10,000 to $20,000</td>
<td>81</td>
<td></td>
</tr>
<tr>
<td>below $10,000</td>
<td>127</td>
<td></td>
</tr>
</tbody>
</table>

**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) 85

**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 80

**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:**

<table>
<thead>
<tr>
<th>Criteria</th>
<th>Score</th>
<th>Rank</th>
</tr>
</thead>
<tbody>
<tr>
<td>Drug D for consideration</td>
<td>190</td>
<td>1st</td>
</tr>
<tr>
<td>Drug A for consideration</td>
<td>178</td>
<td>2nd</td>
</tr>
<tr>
<td>Drug B for consideration</td>
<td>175</td>
<td>3rd</td>
</tr>
<tr>
<td>Drug C for consideration</td>
<td>146</td>
<td>4th</td>
</tr>
</tbody>
</table>
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\textsuperscript{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:\textsuperscript{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 \( \times p_X = \$\text{Drug X} \)) and spending on Drug Y (Drug Y \( \times p_Y = \$\text{Drug Y} \)); i.e. \( \$\text{Drug X} + \$\text{Drug Y} = \text{PHARMAC’s budget.} \)

Thus PHARMAC’s ‘budget constraint’ is illustrated in Figure A1, where the axes are \( \$\text{Drug X} \) and \( \$\text{Drug Y} \) (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 \( \$\text{Drug X} \) and \( \$\text{Drug Y} \) 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

\textsuperscript{25} This is closely adapted from Devlin & Hansen (2000), and the diagrams are virtually identical to ones in Culyer & Wagstaff (1993) and Culyer (1995).
\textsuperscript{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.
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 $\text{Drug X}$ and $\text{Drug Y}$ are shown (the budget line from Figure A1). If you were to choose a particular allocation between $\text{Drug X}$ and $\text{Drug Y}$ 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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