

# PHARMAC's Decision Criteria Summary of Submissions

December 2013



## EXECUTIVE SUMMARY

- PHARMAC issued a consultation document “*PHARMAC’s Decision Criteria*”, in June 2013. The document provided stakeholders with some background to the purpose of the criteria, and information regarding how PHARMAC uses and applies the criteria when making funding decisions. PHARMAC also held regional community forums at twelve locations around New Zealand with over 300 people attending. The consultation document can be found on PHARMAC’s website, at: [http://www.pharmac.health.nz/ckeditor\\_assets/attachments/399/consultation-2013-05-17-decision-criteria-review.pdf](http://www.pharmac.health.nz/ckeditor_assets/attachments/399/consultation-2013-05-17-decision-criteria-review.pdf)
- The closing date for the consultation was 30 August 2013 with a handful of late submissions subsequently accepted. Submissions could be made online through a consultation tool, where submitters could respond to thirteen guiding questions outlined in the consultation document. PHARMAC received 139 submissions, in addition to the discussions recorded at the community forums, and five meetings held with industry and government sector groups. PHARMAC thanks all those who made the time to attend the forums and/or make a submission for this consultation, and appreciates all the feedback received.
- In general most submitters considered that the criteria should be amended to varying degrees. Many submitters noted that the criteria largely work well; however a number criticised PHARMAC for how the criteria are applied in terms of consistency and transparency of funding decisions. Though application of the criteria is more related to process rather than the decision criteria themselves, and therefore beyond the scope of this review, this feedback will be taken into consideration and will contribute to upcoming reviews of other aspects of PHARMAC’s Operating Policies and Procedures (OPP’s).
- Many submitters shared their personal experiences with the decision criteria. A small number reported on positive outcomes; however a large number of submitters reported negative experiences, namely in relation to PHARMAC not funding specific pharmaceuticals. Of these submitters, many commented that they felt the fiscal impact of the funding decision outweighed the consideration of other criteria.
- Many submitters acknowledged the need for criteria to be flexible; this was noted specifically in consideration of rare or complex conditions. However many submitters also felt that a degree of specificity was required to account for discrete areas of PHARMAC’s business, and that criteria need to be objective rather than subjective. A number of people recommended having a broader set of general criteria with layers of specificity below this.
- Submitter opinion was split in relation to the appropriateness of the current criteria for medical devices. It was widely felt that devices were comparatively a lot more complex than pharmaceuticals, and as a result most submitters felt amendment to the criteria would be required to encompass devices. Submitters discussed a number of processes that they felt should be different for devices; including

assessment and prioritisation. Such processes are out of scope of this review but will be included in the medical devices consultation looking into applying the PHARMAC model for medical devices management. Many submitters felt common criteria would be possible if the scope and interpretation of the criteria could accommodate differences.

- Many submitters made recommendations about having an overarching set of principles to preface the criteria. The principles of fairness and equity were discussed by a number of submitters, who felt the criteria need to take into account the health needs of the population as a whole, as well as sub-groups and individuals. It was also stated that criteria should reflect patients' rights, including under New Zealand law and international obligations.
- Most – but not all - submitters considered that the current criteria do not reflect fairness or community values. Submitters largely felt this was due to the lack of explicit reference to fairness or community values in the current criteria. A number of submitters discussed process changes (rather than changes to the criteria) to reflect fairness and community values; for example more stakeholder engagement. However, many submitters also acknowledged that community values are less objective than a set of agreed criteria, different communities have different values, and in some contexts 'community values' may not support particular health services.

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## INTRODUCTION

This summary of submissions reflects the views of stakeholders who responded to PHARMAC's consultation document *PHARMAC's Decision Criteria*, through submission directly to PHARMAC, attendance at the 12 community forums, and/or meetings held directly with PHARMAC staff. This is the first phase of public consultation on PHARMAC's decision criteria. A further consultation on any proposed changes will be undertaken in 2014. PHARMAC thanks all those who took the time to make a submission through the various avenues provided, and appreciates all the feedback received.

### Methodology

PHARMAC released the consultation document on May 2013 on the PHARMAC website, and the consultation period ran until 30 August 2013 with a handful of late submissions subsequently accepted. Written submissions were received from 139 submitters, including state sector agencies, consumer and community groups, clinicians, medical and pharmaceutical groups and individuals. PHARMAC also held meetings with five industry and government sector groups, and held 12 community forums around New Zealand. Further details of the methodology are attached as Appendix 1.

### Structure of Summary

The *Decision Criteria consultation document* posed 13 guiding discussion questions. Most written submissions received by PHARMAC answered the 13 questions, and so this summary is structured according to these questions. For those submissions where general commentary was provided (either instead of answering the questions or in addition to), and where the feedback was received through forums and meetings, this has been incorporated under the most relevant question.

The 13 consultation questions were:

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

## RESPONSES TO CONSULTATION QUESTIONS

### 1. What are your views on the value of the current nine decision criteria

Submitters made general comments about the decision criteria, as well as discussing individual criteria specifically. Overall, comments were related to the criteria as they applied to pharmaceuticals, with few submitters specifically referring to medical devices.

#### *General comments*

Some submitters stated that the current decision criteria are working well or are at least adequate, given that some form of rationing must take place within the context of a fixed budget. Submitters with this view supported the principle of universality, and did not want variable levels of subsidy according to a patient's income, age or disease. One consumer group with this view ('the system isn't broke'), suggested that if any change is needed, it may lie in continuing to increase public understanding of the process and how it works.

Many submitters (professional associations, industry and consumer groups and others) commented on the application of the criteria. These submitters supported the idea that all decisions are assessed according to an explicit set of common measures; however the value of the current criteria was diminished because PHARMAC did not appear to apply the criteria consistently, comprehensively or transparently.

Many submitters considered that the criteria as a whole had not been developed in an appropriate ethical framework. These submitters said that such a framework should reflect New Zealand's human rights commitments, provide equity of access to treatments and involve stakeholder consultation. In particular, these submitters considered that while the criteria meet the needs of the majority they do not meet the needs of those affected with rare disorders.

#### *Individual criteria*

Table 3: some of the key points raised in relation to each individual criterion

Criteria	Summary of submitters views
(1) Health needs of all eligible people within NZ	<ul style="list-style-type: none"> <li>considered to be largely sound</li> <li>clear definition of 'eligible' is required</li> <li>not clear whether it adequately captures or measures the relative significance of 'need' – should include consideration of quality of life (e.g. including patient comfort, burden of side-effects, complicated/inconvenient dosage requirements)</li> </ul>
(2) Particular health needs of Māori and Pacific peoples	<ul style="list-style-type: none"> <li>all eligible people should be treated equally (independent of ethnic group)</li> <li>the particular health needs of Māori and Pacific peoples could be extended to any disadvantaged or vulnerable group</li> <li>obligations under Treaty of Waitangi should be explicit and clarified</li> </ul>
(3) Availability and suitability of existing medicines, therapeutic medical devices and related products and related things	<ul style="list-style-type: none"> <li>use or significance not completely clear – wording could be better expressed</li> <li>suitability needs to be factored in specifically in relation to medical devices); one size does not always cover all</li> </ul>
(4) Clinical benefits and risks of pharmaceuticals	<ul style="list-style-type: none"> <li>analysis of clinical effectiveness essential</li> <li>criteria used to determine clinical benefits are open to debate so PHARMAC must select which advice it prefers – recommended that PHARMAC be more open with what advice it agrees with</li> <li>wider range of evidence should be considered (e.g. experimental treatments, broader consumer benefits)</li> <li>could include long term economic benefits as well as clinical benefits</li> </ul>

(5) Cost-effectiveness of meeting health needs by funding pharmaceuticals rather than using other publicly funded health & disability support services	<ul style="list-style-type: none"> <li>• assessment of cost-effectiveness remains valid</li> <li>• cost analysis should include wider costs to people and society (e.g. burden of disease on patients' family, implications for those living rurally, ability for patient to return to work)</li> <li>• more appropriate to consider cost-effectiveness only relative to other pharmaceutical investments</li> <li>• include therapeutic medical devices and other health care related items (i.e. cost-effectiveness of meeting health needs by funding pharmaceuticals, <i>therapeutic medical devices or other related health care items</i>, rather than other publicly funded etc....)</li> </ul>
(6) Budgetary impact (in terms of the pharmaceutical budget and the Government's overall health budget) of any changes to the Schedule	<ul style="list-style-type: none"> <li>• unavoidable in the context of a fixed budget</li> <li>• should include the direct and indirect costs to patients, their families (and quality of life impacts), DHBs, and the wider health and social services sector – if this was not done under a separate criterion</li> <li>• concern that decisions are too driven by budgetary impacts, which may reduce consumer choice in relation to brand preference</li> <li>• makes it impossible for expensive orphan drugs to be funded</li> </ul>
(7) Direct cost to health user	<ul style="list-style-type: none"> <li>• full significance of this criterion is unclear and amendment and clarity required</li> <li>• 'Wider factors' must be taken into consideration including socio-economic factors, effect on consumer's family, wider societal impact</li> </ul>
(8) 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	<ul style="list-style-type: none"> <li>• should be guided by Government priorities - PHARMAC's decisions could more consistently align with Government health policies, strategies, priorities and targets</li> <li>• too open ended (i.e. in relation to 'or elsewhere')</li> <li>• lacks transparency and therefore not currently helpful - the public have no way of measuring how well or how poorly PHARMAC is delivering on the Government's health priorities through its funding decisions</li> </ul>
(9) Such other criteria as PHARMAC thinks fit.	<ul style="list-style-type: none"> <li>• too vague and lacks transparency in when/how applied</li> <li>• important as one size does not fit all and enables a subjective approach as required</li> </ul>

## 2. What have been your experiences with our current decision criteria?

A small number of submitters (individuals, a consumer group and clinical professional associations) stated that their experiences had been largely positive. These submitters had found the decision criteria to be largely fair – with the benefits outweighing the problems.

A large proportion of submitters, including industry and consumer groups and individuals, and attendees at community forums detailed difficulties with the application of the decision criteria. Most of these respondents referenced funding decisions made by PHARMAC on specific pharmaceuticals.

### *Lack of transparency in the process*

The primary complaint from submitters who reported negative experiences with the criteria was the lack of transparency in how and when the criteria were applied. Overall, these submitters considered that:

- PHARMAC does not provide any explanation of how applications have been assessed under the criteria either at the consultation stage or once the decision has been notified;
- many/most applications appear not to be presented to the PHARMAC board (or otherwise considered for acceptance or decline)
- the relative importance of each criterion remains obscure;

- while bound by Government policies, priorities and targets, PHARMAC does not have to justify any decision that is not consistent with these goals; and
- timelines are not clear.

#### *Unclear application of decision criteria in process*

Industry and consumer groups in particular expressed confusion and recommended more clarity in regard to how the criteria are applied (and by whom) at different stages of the decision making process. The issue of how the Pharmacology and Therapeutics Advisory Committee (PTAC) used the criteria was specifically mentioned by a number of submitters. Of these submitters, many referenced the PTAC minutes, and it was noted that generally only three or four of the nine criteria are considered. In addition, the short summary at the end of the minutes implies that the criteria 'do not drive or frame the discussion.'

Further, one submitter considered that 'no weighting is ever given to the use of the nine criteria and any examination of decisions made by PHARMAC cannot determine the extent to which one criteria has dominated or been undermined by other criteria.'

#### *Fiscal impact eclipses all other criteria*

A number of submitters considered that criterion 6 (budgetary impact) carried the most weight for PHARMAC. In addition, PHARMAC's judgement of cost-effectiveness was thought to be made only in relation to the impact on PHARMAC's budget, not on the whole health sector or total cost to society. Some submitters stated there was no indication that human rights (expressed in the Human Rights Act 1993 and a number of United Nations conventions that New Zealand is a signatory to), or certain matters covered by the New Zealand Public Health & Disability Act 2000, the New Zealand Health Strategy, or the Medicine Strategy, are given consideration. One submitter claimed that PHARMAC was not currently meeting its legal obligations in this regard.

#### *Experiences with specific pharmaceuticals*

Consumer groups and some individuals extensively described largely negative experiences with specific pharmaceuticals not currently listed on the Pharmaceutical Schedule, or PHARMAC's decision to fund a particular type of pharmaceutical or technology only. This included:

- several contraceptive devices not currently listed: Cerazette, Implanon, and in particular the Mirena intrauterine system which is not listed for contraception;
- PHARMAC's decision to provide only the CareSens brand of blood glucose testing meters;
- eculizumab (Soliris) which is not currently listed for paroxysmal nocturnal haemoglobinuria (PNH);
- alglucosidase alpha (Myozyme) which is not currently listed in the treatment of Pompe disease;
- the replacement of Inhibace with the generic Cilazapril;
- calcort (Deflazacort) which is not currently listed for the treatment of Duchenne muscular dystrophy; and
- lack of consideration of Special Foods in the decision making process.

### **3. To what extent should the criteria give PHARMAC the flexibility to make decisions on a case-by-case basis, and to exercise judgement?**

Most consumers submitting on the topic of case by case judgement emphasised the need for flexibility in the cases of rare and complex conditions, particularly where patients face a



poor prognosis for survival or prolonged pain and suffering without access to the treatment or device. Many of these submitters further stated that any judgement exercised needs to be in consultation with affected consumers.

Conversely a number of submitters also emphasised the risk arising from too much flexibility. For example, if flexibility becomes too much part of the process, the objectivity of PHARMAC may be lost and there will be opportunities for political pressure to interfere with decision making. Some submitters considered that flexibility already existed within PHARMAC's decision making processes through Special Authorities, the Named Patient Pharmaceutical Assessment (NPPA) and criteria 7 and 9.

Submitters recommended PHARMAC could consider the following approaches to exercise flexibility appropriately:

- *Separate fund for special cases:* to enable consideration on a case by case basis for situations such as high cost orphan drugs, or a separate scheme all together, such as those implemented in other countries (e.g. England)
- *Supporting criteria with guiding principles:* this would establish the usual limits of the criteria and define when it is acceptable for PHARMAC to step outside such limits. The decision criteria would also outline the process that PHARMAC must follow in doing so.
- *Different tiers of decision making:* decision criteria should be broad enough to encompass the scope of all decisions made by PHARMAC. More specific (and multiple) criteria) could then be defined at a lower level. This would enable more flexibility.
- *Using existing criteria more transparently:* could include specific consultation on, or justification for, any exceptions to the accepted definition, interpretation or weighting of any criteria.

#### **4. Is there anything about the nine existing criteria that make them inappropriate to be applied to medical devices? Why?**

Submitter opinion was fairly evenly divided as to whether the existing criteria are appropriate for medical devices. However, it should be noted that some submitters considered the criteria appropriate for medical devices with amendments, while others considered the criteria inappropriate because they would require amendment.

##### *Minor amendments to incorporate devices into existing criteria*

Of those submitters who considered that the existing criteria are appropriate to use with medical devices, many noted that minor amendments were needed to make it explicit that the criteria also referred to medical devices. An industry submitter noted that the application of the criteria may differ between devices and medicines. However, this submitter noted that this may be more process-related, and the differences in the application could be explained through supporting documentation such as the Guidelines for Funding Applications to PHARMAC.

##### *Devices are fundamentally different to pharmaceuticals*

Various submitters across different submitter-types considered the current criteria inappropriate, given devices are fundamentally different. Key differences included:

- substantive support services required for new devices (e.g. administration, technical support, education and training requirements);
- patient outcomes associated with the medical device depend on the device, and on clinician skill and procedural technique (the latter likely to improve with experience);
- medical devices involve capital expenditure and maintenance;

- more direct costs to DHBs; including inventory, human resource implications, outsourcing requirements etc;
- lifecycle of medical devices is significantly shorter than pharmaceuticals;
- many medical devices are customised for patient anatomy, physiology and dynamics, thus there is a need for a range of similar alternatives; and
- devices requiring implantation (or prostheses) may require revision, which necessitates ongoing supply of the device, as well as continuity of clinician expertise.

#### *Inappropriateness of criterion 5*

Several submitters (a state sector agency, a professional association and an industry submitter) specifically noted the inappropriateness of the current criteria 5 (cost-effectiveness) for medical devices. This was namely in relation to the additional costs that need to be taken into consideration some of which are noted above. Some submitters also considered that while cost effectiveness is a useful criterion, in some situations survivability of the device, cost of revisions and surgical skill would have to take precedence.

#### *Differences in overall assessment of devices*

Generating sufficient evidence to inform decision making was noted as a major challenge for most medical device manufacturers, given the costs incurred and time involved in generating data. Additionally, it is often the case that trial data are not available. A number of submitters therefore recommended clinical evaluation be based on considerations other than randomised controlled trials for example clinical opinion, case studies and observational data. Several industry groups and professional associations suggested specialist advice may be required when considering funding applications for devices. One professional association suggested that PHARMAC create an assessment framework that includes local and international evidence and feedback on particular devices.

Timing of assessment was considered by several submitters to be one of the most important differences between devices and pharmaceuticals. Medical device technology can be obsolete in a very short period so any assessment and prioritisation process needs to keep up with the rate of change. Delays to decision making will mean that products become obsolete before introduction into the market.

#### *Other associated risks*

An industry submitter and a professional association considered that implementation of the current decision criteria would not acknowledge the importance of change management in relation to medical devices. That is, the cost of change to the patient, the health risks associated with the change and the societal impact of the change.

Another submitter also stated that the innovation cycle of medical devices needs to be considered as there is a major difference between bringing a medical device to market in comparison to a pharmaceutical. Having a limited device catalogue and slow decision making on what devices are to be funded, especially new and innovative ones, has consequences on the social capital of the orthopaedic workforce and physician retention in New Zealand.

### **5. What other criteria might be needed when considering the priority of a medical device?**

As with the previous consultation question, there was some difference in presentation as to whether the considerations submitters wanted to see expressed constituted additional criteria or amendment to current criteria.

a. *Cost*

A number of submitters considered that overall health economics should be included in the decision making process and not solely the initial purchase cost of any device. (As it was not clear to some submitters whether total costs could be assessed under the existing criteria, additional criteria may be needed.) Overall, submitters considered that the assessment and prioritisation process should take a long term economic view with the total cost of the care pathway and the total cost of products being taken into consideration, as well as direct and indirect patient and other stakeholder benefits (whānau, carers, community) being taken into account.

Several of these submitters referred to PHARMAC's discussion of this matter in *Submissions analysis: PHARMAC and hospital medical devices – Obtaining clinical input*. Costs to be considered were also mentioned in response to question 4 regarding fundamental differences between devices and pharmaceuticals (listed on page 5). Additional costs mentioned include: consumables, compatibility of device with the clinical environment, expected life of the product, and upgrade pathways and flexibility with technology advances.

b. *Clinical safety*

Several submitters commented on the need to have criteria in relation to the clinical safety of medical devices, with some stating that a focus on patient safety and the delivery of high quality healthcare services should be overriding considerations. PHARMAC was advised by submitters to consider risk classifications when prioritising medical devices as the various risk classes will require different criteria of assessment and have different timeframes.

In particular, several submitters noted the lack of premarket regulatory approval process, other than a notification to the Web Assisted Notification of Devices (WAND) database, for medical devices in New Zealand. These submitters stated that PHARMAC must consider the safety of medical devices, or have in place a process that enables it to make recommendations which take into account clinical safety.

c. *Usability*

Some submitters and a number of attendees at community forums considered usability of the device needed to be a criterion. This included consideration of factors such as the ease of use (for clinicians and patients), how much additional training was required, and how quickly clinicians can safely and efficiently use a new device.

d. *Level of need*

Several submitters suggested that level of need be part of the criteria. One consumer group considered that at the individual client level the degree of need for the device, the forecasted health improvement and the financial circumstances of the client should be criteria.

e. *Assessment in relation to the supplier*

As noted, a number of submitters commented on the relevance of support and services offered by device suppliers, including technical training of surgeons and clinical staff, ongoing professional education, and in-theatre support where required. One submitter suggested that PHARMAC should consider the ability of the medical device supplier to provide the necessary support. One group considered that company size or supplier history should *not* be a decision criterion. Rather, when appropriate, PHARMAC should support multiple suppliers to encourage competition and innovation – taking into account supplier quality, product support and supply issues.

f. *Disinvestment decisions*

Several submitters referred to criteria for making disinvestment decisions relating to medical devices. One industry submitter stated that the current criteria are either redundant or deficient in a number of respects for the purposes of assessing disinvestment decisions.

*g. Different considerations for vaccines*

PHARMAC did not seek specific feedback on the criteria for vaccines; however, several submitters suggested that some new considerations need to be incorporated into the criteria to ensure that PHARMAC's assessment of vaccines is accurate and comprehensive. This included factors such as:

- consideration of vaccines being preventative (versus treatment based);
- greater emphasis on acceptability to patients and health professionals (to encourage better uptake);
- public health considerations may take precedence;
- differences in clinical evidence may be required; and
- separate expertise may be required to advise on efficacy, safety, implementation and evaluation.

**6. What advantages and disadvantages would there be in all PHARMAC's decisions, for pharmaceuticals and devices, being made using the same criteria?**

Most submitters who commented saw both advantages and disadvantages for decisions made using the same set of criteria for both pharmaceuticals and devices. Most points raised by submitters were also discussed in detail in the previous two questions.

***Advantages***

*Comparative allocation of limited resources*

The primary advantage seen by various submitters (including consumers, industry and state sector agencies) was that a common set of criteria best provides for the comparative allocation of limited resources regardless of the treatment model applied. This would also help to assure stakeholders that all interventions, regardless of their nature, were being given full, fair and equal consideration by PHARMAC.

Several consumer groups pointed to the advantage of consistency: all people suffering a condition would receive the same level of access to pharmaceuticals or devices no matter where they lived in New Zealand. There was therefore an inherent sense of fairness in being able to apply the same set of criteria across all spending. Some submitters acknowledged this may be difficult in practice and recommended there should be the option to use variations or category specific criteria in special cases. Variations should be acknowledged in the decision making framework and reported in a transparent manner.

*Comparison of cost/benefit data for pharmaceuticals and devices*

A few submitters noted that an advantage of having common criteria was that the existing criteria are tried and tested, with efficiency outcomes known. Further to this point, several submitters considered that with the same criteria, cost/benefit data (including information on wastage) could be directly compared. It was noted by these submitters that key differences between pharmaceuticals and devices must be acknowledged in any such analysis, for example, the long life of medical devices and the requirement for industry support throughout the life of the device.

*Accommodating differences within common criteria*

An industry submitter considered that PHARMAC could adopt common decision criteria for investment in pharmaceuticals and medical devices if the scope and interpretation of the criteria accommodated differences between the two business areas. Related to this point, several industry groups noted the potential disadvantage this might create, in that the criteria would remain broad or even require greater flexibility. However, this situation would be

acceptable provided that different importance is assigned to particular decision criteria and consistent and transparent processes are implemented.

A state sector agency also noted that using the same criteria does not imply that the analysis for medical devices and pharmaceuticals must be identical.

### ***Disadvantages***

Two industry groups stated that their experience has shown that the preferred criteria for selection of medicines and medical devices differ greatly among funders, physicians, patients, communities and policymakers. These submitters felt that using the same set of criteria for both would jeopardise specificity and clarity and ultimately affect patient outcomes.

#### *Comparative complexity of devices*

As discussed previously, many submitters (a state sector agency, consumer groups and industry) considered that medical devices required a more complicated and multifaceted approach than pharmaceuticals. These submitters did not consider that the current decision criteria could necessarily address the comparative complexity of medical devices, and the different evaluative methods required (as discussed in the question 5). Consequently, while there may be overlap in some of the decision criteria, there needs to be a number of separate decision criteria.

Related to complexity, one group questioned whether PHARMAC intended to bundle devices with pharmaceuticals in contracts. This submitter noted that bundling would be an advantage to companies who have both pharmaceutical and medical device divisions.

## **7. How specific or general should the criteria be?**

Of those who specifically commented, submitter opinion was approximately evenly divided between the criteria being as general as possible and the criteria being as specific as possible. Responses depended to some extent on the level of detail submitters considered could be in the criteria, and whether there was an overarching set of guiding principles applied to the criteria. This discussion reflected that in response to question 3 (flexibility).

### ***Criteria should be as general as possible***

Industry and consumer groups noted that the current criteria show that general criteria can provide an adequate framework for the assessment of most proposals. It was preferable that all product categories for which PHARMAC is responsible should, and can, fit within the same set of decision criteria. Key rationales outlined by submitters for maintaining general criteria are discussed below.

#### *Criteria should offer guidance not prescription*

Submitters supporting the criteria being as general as possible considered that the purpose of the criteria is to offer guidance and not prescription, and therefore the criteria should be useful and concise. In addition, tools used in deciding what to fund need to be simple and systematic (such as Quality Adjusted Life Years divided by cost) in order to be dispassionate (and avoid a myriad of special case decision-making influenced by individuals, families, physicians and the media), diminish political interference, increase consistency, and be efficient because a complex process will not be economic, and may be misunderstood or misinterpreted.

#### *Criteria should be flexible*

Submitters across submitter-type considered that overall, a common set of general criteria provided the opportunity for adequate flexibility better than multiple sets of specific criteria – provided the criteria were applied in a transparent manner. One consumer group noted that

flexibility is needed to ensure all factors are considered, even if this comes with a risk of generating some inconsistencies between different decisions. An industry submitter suggested some improvements to the process to make it more flexible could include: implementing a ranking score for each criterion, providing an accompanying definition for each criterion, and writing the criteria more clearly to make them easy to interpret.

#### *Specificity could occur at the next level down*

As noted in response to question 3, a number of submitters thought specificity could occur at the next level down. In this way, decisions in relation to particular categories (devices, medicines and – for some submitters – vaccines, rare disorder treatments and NPPA applications) can have the same general decision making criteria but differences in the application of the criteria can be specified at a lower level. A couple of submitters noted this additional information could be captured in The Guidelines for Funding Applications to PHARMAC and the Prescription for Pharmacoeconomic Analysis (PFPA) publications.

#### **Criteria should be as specific as possible**

##### *Providing greater clarity and transparency*

Of those submitters recommending more specificity (largely industry and consumer groups), the main rationale was to provide more clarity and transparency around decision making and ultimately transparency of the assessment and purchasing process. As with those submitters supporting very general criteria, those wanting specific criteria also suggested that the criteria should have an accompanying definition or supporting description linked to them to ensure they are adequately clear.

##### *Taking account of complex data*

A consumer group noted that in assessing funding applications PHARMAC has to take into account a great deal of complex data. If the criteria are too general, decisions are more likely to be based on the judgement call of PHARMAC staff than on a robust, fair and transparent analysis.

## **8. What other criteria should/could PHARMAC consider?**

### **General considerations**

Submitters across different submitter-types suggested a number of general considerations that should apply to whatever set of criteria is used.

- Consistency in decision making – in relation to access to treatment across diseases (for the same level of severity), across patients and across time
- Reflect prevailing community values – to support fair and equitable distribution of health resources across all patient groups (discussed further in response to question 12)
- Incorporate consumer input – to ensure a consumer-centred approach
- Ensure legal obligations are met under applicable New Zealand and international law and conventions, and obligations are met in the wider health care sector, including in relation to DHB's obligations towards patients; and
- principles of fairness and equity be reintroduced to PHARMAC's OPPs documents.

#### *Orphan drugs and the treatment of rare diseases*

Related to the above themes of legal obligations and ethical values, many submitters (consumer and community groups and individual consumers) commented on how the current criteria adversely affect the chances of high cost and orphan drugs being funded.

Submitters believed more weighting should be given to patients with rare diseases through additional criteria or differentiated criteria under NPPA. One submitter noted that a recent

report by the Ombudsman supported decision criteria under the NPPA policy being clearly differentiated from those used for making listing decisions on the Pharmaceutical Schedule.

Submitters proposed various approaches to improve consideration for small populations or individuals including: an additional layer of decision-making for very rare diseases that do not fit standard cost-effectiveness thresholds for large populations; a weighting built into decision criteria to counter the disadvantage of rarity; a ring-fenced allocation of funds from the Government for orphan drugs; another agency to manage individual claims; adding high cost drugs for rare diseases to the Schedule with the cost met by patient contributions across all medicines, except where patients are unable to contribute.

### ***Suggested new criteria***

Submitters across interest groups suggested a range of considerations PHARMAC should incorporate into their decision making – either as new criteria or as explicit considerations within the existing criteria. In general, these suggestions were applicable to both pharmaceuticals and devices.

#### ***a. Health need for all New Zealanders***

It was widely considered that appropriate medicines and devices should be available for all New Zealanders inclusively – and illnesses should not be treated as a particular problem for any one group such as might be defined by ethnicity, age or financial circumstances. Related to this, a number of submitters specifically rejected the addition of any criteria that considered certain circumstances, such as the whether a condition was self-inflicted. For example, a physician opposed any decision on treatment for diseases being based on consideration of how ‘preventable’ they are. Conversely, other submitters considered that self-inflicted medical conditions should not be eligible for funded treatment.

#### ***b. Consideration of health needs of specific interest groups***

There were a few submitters that considered the health needs of Māori and Pacific peoples needed to be particularly addressed both as an expectation of the Treaty of Waitangi (in the case of Māori) and also to recognise the health inequalities that exist between the health of Māori and Pacific peoples and other eligible New Zealanders.

A small number of submitters considered that the particular health needs of children should be prioritised. One submitter’s rationale for this suggestion was New Zealand’s Human Rights obligations under the United Nations Convention on the Rights of the Child (relating to the prioritisation of treatments for children).

#### ***c. Severity of condition***

A few submitters recommended that PHARMAC explicitly consider including the severity of condition within its decision criteria.

#### ***d. Product safety***

A small number of submitters (including professional associations, a state sector agency, consumer groups and individuals) stressed that product safety should be a criterion. For example, a criterion might be ‘any risks associated with the product (including supply risk) and/or the quality of evidence behind it that warrant caution.’ A small number of submitters considered this should be a criteria specific for medical devices as well.

#### ***e. Clinical effectiveness and acceptability***

Some consumer groups and consumers stressed the importance of ensuring scientific rigour in relation to clinical effectiveness. Submitters also considered the need to take in a wider range of evidence in relation to effectiveness such as experimental treatments. Relatedly, several clinical and industry submitters recommended that clinical acceptability be a criterion, that is, the practical and clinical impacts on clinicians. This was discussed in relation to both medical devices and pharmaceuticals.

*f. Public health considerations*

Several submitters suggested that public health considerations be a criterion or an extension to criterion 4. For example the criterion might be termed 'The clinical benefits and risks of pharmaceuticals to the recipient, their family, and the wider community' - extending the benefits and risks of pharmaceuticals beyond a recipient to their family and the community as a whole. Submitters considered this particularly relevant in the context of communicable diseases where treating a patient and their contacts reduces the potential and size of outbreaks and therefore the cost to the health system.

*g. Price*

One industry submitter noted that PHARMAC currently takes price into account in making decisions (eg, in the tendering process), and therefore needs to be an explicit decision criterion, rather than being considered implicitly under 'budgetary impact.'

*h. Costs*

Submitters asked for criteria that enabled PHARMAC to take a broader consideration of costs (financial and non-financial) than exists currently; for example, costs to the patient's family, impact on individuals' wellbeing and ability to manage their condition, ability to contribute to society etc.

Some submitters also thought government priorities (non-health) should be considered part of PHARMAC's fiscal responsibility as budgetary management is on behalf of the taxpayers of New Zealand and should not be narrowly focussed on the health budget. It was noted that some medicines, vaccines and devices have considerable impact on non-health spending by government.

A professional association and industry groups recommended that PHARMAC include the direct and indirect costs to DHBs as decision criteria, for example hospital costs, enhanced productivity, efficiencies and performance.

*i. Impact on the environment*

A professional association of health professionals in New Zealand and several individual submitters noted their concern about the impact of global climate change on health and health services. These submitters requested that PHARMAC include in its decision criteria the impact on the environment of subsidising any specific drug or device, and asked that it consider whether there are alternative products or companies that have a lower environmental impact.

Related to environmental impact, groups and individuals, including a pharmacist, commented on the need for the impact of both economic and environmental waste to be part of the decision criteria. These submitters suggested that suppliers be asked to account for the costs of wastage, such as medicines disposal, in their tender bids.

**9. Of the current criteria, which remain appropriate to retain? Why? Which ones are no longer appropriate? Why?**

Relatively few submitters directly responded to this question, with many referring to their answers given to question 1. Any additional feedback in relation to specific criteria has therefore been included into Table 3 (page 3).

Table 5 shows that of those submitters who did directly respond to this question, retaining all of the current criteria was supported by a majority (as shown in Table 5). Submitters' views about retaining criteria were influenced by whether from their perspective additional considerations could be added to existing criteria or whether this would constitute different criteria.



Decision Criteria	No longer appropriate	Should be retained
1	2	14
2	5	11
3	2	13
4	1	13
5	3	13
6	3	13
7	3	11
8	4	11
9	5	9

#### *Application of the criteria*

Many submitters across interest groups commented on the application of the criteria generally, stating that the most important factor was that the criteria are applied consistently and transparently. Some of these submitters supported adding specific information within the criteria to minimise misinterpretation: this could be in the form of a set of guiding principles stated with the criteria.

Additionally, industry groups suggested that PHARMAC's processes would be greatly improved if it consistently framed all published materials (minutes, consultations and notifications) in terms of the decision criteria and could, in any other way, provide assurance that all applications are properly being assessed according to the decision criteria. An industry group also submitted that PHARMAC should not include criteria that are effectively redundant in the decision making process.

#### **10. If you were to have a clean slate, around which criteria would you base decisions for funding pharmaceuticals within a fixed budget?**

Several submitters – professional associations and consumer groups – reiterated that the current criteria should remain, with some additions. Similarly, industry groups generally considered that the current criteria are largely appropriate but could be amended to better reflect what PHARMAC appears to actually take into account.

Several submitters suggested PHARMAC look to international models of best practice. The Ontario Health Technology Advisory Committee was one example suggested, used for both pharmaceuticals and devices.

#### *Guiding principles*

As mentioned in response to other questions, many industry submitters, and some professional associations and consumer groups recommended prefacing the decision criteria with some guiding principles (which would need to be included in PHARMAC's OPPs). Such a set might include general principles (such as transparency and fairness) as well as further explanation or examples of the commonly applied interpretation of each of the criteria.

Several of these submitters noted that in the first edition of the OPPs there were explanatory notes on where and how the decision criteria were intended to be used. Submitters considered that the OPPs should reflect the original intention that PHARMAC is supposed to apply its decision criteria to all funding applications.

### *Suggested criteria*

Suggested criteria in response to this question included:

- Stated Government health priorities
- consistency with legal obligations
- equity
- impact on health need
- clinical benefit, safety and effectiveness
- value for money
- direct cost to health service users and disease burden
- budgetary impact
- feasibility of adoption (a component of the Ontario Health Technology Advisory Committee and the Queensland Policy and Advisory Committee on New Technology)
- impacts on health professionals
- other criteria as PHARMAC thinks fit
- other matters influencing the decision (including political, commercial, immediate or long-term considerations.)

### **11. How do the current criteria reflect fairness or community values?**

Several submitters responding to this question acknowledged that PHARMAC has a very challenging task. However, amongst submitters commenting on this question, most considered that the current decision criteria do not reflect fairness or community values.

#### *No explicit reference to fairness or community values*

Across interest groups, many submitters considered that the current criteria make no explicit reference to fairness or community values – apart from acknowledging the health needs of all eligible people within New Zealand and specifically identifying Māori and Pacific peoples. Further, the criteria do not direct PHARMAC to adequately consider the social impact of a funding decision.

These submitters considered that the current criteria therefore need to be changed. If the social impact was considered, PHARMAC would be better able to decide whether a decision to fund or not is fair and could better understand the impact on a community by considering how individuals and families will be affected.

Several submitters stated that community values (such as the rule of rescue) support fair and equitable distribution of health resources across all patient groups. The criteria should therefore support:

- the human right of everyone to the enjoyment of the highest attainable standard of physical and mental health
- the right of each person to share in the benefits of scientific progress (Article 15 of the Universal Declaration on Human Rights)
- the right of a person to a standard of living adequate for health and well-being, and
- the right to security in the event of sickness, disability or other lack of livelihood in circumstances beyond a patient's control.

Two submitters noted that even though such values are challenging to define, it is not acceptable for them to be ignored in decision making, or left to the discretion of those making the decisions.

### *Current criteria do reflect fairness*

Several submitters, including a professional association and a clinician, stated that the current criteria broadly reflect fairness. One submitter considered that PHARMAC has taken account of the many facets funding decisions are required to consider. This submitter agreed with the article written by Professor Anthony Harris in PHARMAC's consultation document, outlining that funding decisions work best when based on a set of principles and considered that PHARMAC's QALY system along with comparison and prioritisation provides a good system for making decisions. Considering factors such as earning potential was regarded as a 'slippery slope.'

## **General concerns**

### *Consultation*

Many submitters (professional associations, industry groups and consumers) commented on the necessity for PHARMAC to consult with stakeholders. These submitters noted that overall there was no indication in PHARMAC's past practices or policies that consumer engagement is given any more than 'token consideration' when actual funding decisions are contemplated.

Some of these submitters suggested PHARMAC's effort to engage with local communities as part of the consultation process was not a sustainable method long term for incorporating community values into the decision making process. These submitters suggested PHARMAC should formally survey (or use a citizen panel) to establish a meaningful way to incorporate fairness and community values into its decision framework, in a timely manner.

### *No commonly accepted conception of fairness or community values*

A number of submitters noted that community values are less objective than a set of agreed criteria. Expressions of community morality are influenced by gender, ethnicity, age and ability and therefore reflect the views of some groups and not others. Additionally, different communities may have different values, which may change over time, making this problematic to include in a specific criterion. One community group noted that in some contexts 'community values' would not support particular health services being offered, for example, safe and accessible abortion services, and consequently certain groups would be disadvantaged by the inclusion of a community values criterion.

### *Equity rather than fairness*

Several consumer and community groups considered equity a better outcome than fairness as giving people the same treatment does not improve outcomes for certain groups such as those suffering rare diseases or those on low-incomes. A system that favours particular groups such as those on low-incomes should result in more even health outcomes, which leads to equity.

### *Process rather than criteria require amendment*

A number of industry and consumer groups submitted that PHARMAC's processes rather than the criteria required amendment in order to be fair. These submitters stated that perceptions of fairness rely on decisions being reasonable and justified. Consequently, PHARMAC's processes, rather than the decision criteria need amendment in order to reflect fairness and community values. A number of submitters noted specifically that the lack of published information about decisions made should be more readily available in the interests of being fair and transparent.

## 12. What additional criteria would you suggest to reflect fairness or community values and how could these be measured?

Most responses to this question focused on the additional criteria rather than measurement. Several submitters made comments about the overarching principles and changes to process that PHARMAC should apply to reflect fairness and community values in the decision making processes. These have largely been discussed in detail in response to earlier questions. This included;

- New Zealand's legal obligations and human rights framework
- Systematic and formal inclusion of consumer and community views across the decision making processes
- More consistent and transparent use of the criteria
- Community values - examples included: those who can, pay more; environmental protection; rural impact.

### **Additional criteria**

The following additional criteria were suggested by some submitters to support commonly held social values – and contested by others.

*Equity and health needs of particular groups:* A number of groups suggested adding a specific equity criterion. This criterion could be used to ensure the health needs of particular groups, such as those with life-limiting rare disorders and children were properly considered. Alternatively it was suggested that the consideration of New Zealanders' health needs criterion and the particular health needs of Māori and Pacific peoples' criterion could be amalgamated under one equity criterion.

Some submitters considered equity was already addressed by PHARMAC. Other submitters were against the concept of prioritising the needs of particular groups on the grounds of race, disability, age, gender, sexual orientation, religion, beliefs, or socioeconomic status. These submitters considered no group should be afforded superior access where this would deny or reduce access for persons not members of those groups.

*Quality of life:* Some submitters considered quality of life should be the focus of decision making, together with known survival rates for the particular disease with the use of appropriate medicines (thus following National Health Institute for Health and Care Excellence (NICE) principles). Submitters also suggested there should be more active review of when treatments for patients near end-of-life stage are futile and should be withdrawn.

*Unmet clinical need:* It was suggested by several submitters that PHARMAC should consider unmet need from an individual patient or patient group perspective – rather than considering the number of products funded for a condition, as it does now.

*Personal responsibility:* Several individual submitters commented on the need for a criterion that reflected the role of personal responsibility in good health. However, a number of submitters emphasised it was not appropriate for PHARMAC, or health institutions in general, to make value judgements about the lifestyle or behaviour of individuals when making decisions regarding access to healthcare.

*Broader socioeconomic benefits:* A number of submitters across interest groups suggested the criteria focus on the broader socioeconomic benefits of funding (and not funding treatment), including the indirect costs across other government spending. One submitter noted the importance of having such a 'total costs' criterion given the role that innovative medical devices and other medical information technology is increasingly having.

*Proven effectiveness of medication:* A number of industry and consumer groups supported a new criterion of the proven effectiveness of medication. Under this criterion, if a medication

is found to be ineffective for the condition for which it was originally prescribed it should be replaced by a better one.

*Additional criteria for medical devices:* A number of submitters across interest groups suggested that the safety of medication and devices be a criterion. One submitter suggested a number of additional criteria which would reflect community values in relation to devices. This included; availability and suitability of medical devices with acknowledged clinical specialist input, flexibility according to patient appropriate needs, impact and implementation of technology on clinical practice and patient behaviours, and negative health impacts of denying access.

### **Measurement**

Several submitters noted that measurement of fairness or reflection of community values is more difficult than other matters often dealt with by pharmacoeconomic analysis, but the fact they are difficult is not a justification for avoiding them. It was noted that economic analysis also depends on the particular inputs and assumptions that are made in the system.

A few submitters commented on the range of social impact assessment tools available, with one noting that other parts of the health system have a variety of tools for measuring health status of different populations by age, sex, socio-economic status, deprivation, ethnicity, and so on. Many of these tools could be explored for application to patient groups in need of pharmaceuticals. One submitter suggested that PHARMAC take advice from the market research sector on how decision criteria can be ranked and measured.

However, one submitter commented extensively on the findings of four literature reviews on public preferences for the allocation of healthcare that have been published, and suggested Hansen's (2006) review of PHARMAC's approach to funding pharmaceuticals is still applicable.<sup>1</sup> That is, there is no generally accepted method of incorporating fairness and community values into health technology assessment.

### **13. What additional information or detail do you think should be included in the decision criteria section of the OPPs?**

#### *A set of guiding principles*

Most industry submitters and some consumer groups and professional associations responding to this question suggested that the criteria would be improved as a tool if they were supported, as they used to be, in the OPPs by a set of guiding principles that reflect how PHARMAC intend to apply their decision criteria. Such a set might include general principles (such as transparency and fairness) as well as further explanation or examples of the commonly applied interpretation of each of the criteria.

Submitters considered that having such a set of principles would aid transparency, better ensure that PHARMAC's consultation and notification documents explain proposals and decisions in terms of the decision criteria, and ensure greater consistency in the application of the criteria.

In relation to transparency, one submitter suggested a rating scale which allows interested parties to understand how a decision was reached by explaining which decision criteria were considered the most important and relevant would be desirable. This could be published in a Public Summary Document.

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<sup>1</sup> Hansen P (2006) *A theoretical review of PHARMAC's over-arching approach to deciding which pharmaceuticals to fund, including high cost ones*; Department of Economics, University of Otago, Dunedin, New Zealand

### ***Other information for the OPPs***

*Clarification of the review process:* Consumers groups and industry submitters suggested that the process for appeal and review could be clarified in the OPPs.

*Non-schedule applications:* A consumer group noted that the current criteria are difficult to apply where the decision in question is not a Schedule application. This group suggested that the OPPs state the criteria for each of the decisions PHARMAC makes, rather than a general statement about endeavouring to apply the criteria to the extent that they can be applied. In the separate statements it would also be appropriate to clarify any policies for weighting of the criteria.

*Specification of the criteria that apply to pharmaceuticals and/or devices:* A professional association suggested that the Decision Criteria section of the OPPs (Section 2.2) clearly specify which criteria apply to pharmaceuticals and devices – or that all criteria can be applied to either. The provision of a brief explanation and some examples of the kinds of issues that may be considered under each criterion was considered by this submitter to be particularly important for devices.

*Establishing decision criteria in relation to new areas of business:* Two industry submitters suggested that PHARMAC's OPPs need to contain procedures for establishing decision criteria in relation to new areas of business. Such a procedure should require PHARMAC to complete consultation on the appropriateness of the proposed criteria before they are utilised in any commercial context.

## Appendix 1

### Methodology

PHARMAC received 139 submissions in response to the decision criteria consultation document. The types of submitters are included in Table 1 below. PHARMAC also held five meetings with various industry and government sector groups, and 12 community fora around New Zealand. Table 2 outlines the number of attendees at each forum. The notes from these meetings have been included in the analysis.

**Table 1. Description of submitters**

Type of submitter	No. of submissions
Clinician/Prescriber	10
Consumer	48
Other individual	11
Pharmacist	3
State sector agencies	3
Consumer Group	27
Pharmaceutical/medical device industry	9
Professional association	20
Other group	8
<b>Total</b>	<b>139</b>

**Table 2. Community fora**

Community fora	Number attending
Auckland – Central	43
Auckland – South	7
Christchurch	32
Dunedin	44
Hamilton	30
Hokitika	25
Nelson	34
New Plymouth	6
Palmerston North	14
Tauranga	44
Wellington	27
Whangarei	18
<b>Total</b>	<b>324</b>

### Preparing the summary of submissions

Submitters' responses were entered into a database, using a coding framework developed from the questions presented in the consultation document. In the analysis, emphasis has been placed on the range of views presented, rather than on the numbers of respondents expressing a particular view. Counting was difficult because some of the responses represented a single voice, while others represented several or many people. For this reason, the number of submitters responding to each question in the consultation document has not been provided. An indication of the level of support for various positions has been given in places to show how widely held particular views were.

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