

# PHARMAC's Decision Criteria Proposal for Change

Summary of Submissions

July 2014



## Executive Summary

- PHARMAC issued a consultation document, [PHARMAC's Decision Criteria: Proposal for Change](http://www.pharmac.health.nz/about/operating-policies-and-procedures/decision-criteria-consultation), in February 2014. This consultation document outlined PHARMAC's proposed changes to the Decision Criteria that were developed following the first round of consultation carried out in May-August 2013. More information on the consultation process to date can be found on the PHARMAC website: <http://www.pharmac.health.nz/about/operating-policies-and-procedures/decision-criteria-consultation>.
- The closing date for feedback on the consultation document was 21 April 2014, with a handful of late submissions accepted after this date. Forty nine written submissions were received and one meeting was held on request with a stakeholder group. A wide range of stakeholders made written submissions, including consumer groups, District Health Boards (DHBs) and clinicians, industry representatives, professional associations, members of the public and state sector agencies. Feedback was also gathered through a half-day Stakeholder Consultation Event, a meeting with Māori stakeholders, and through some of PHARMAC's regular stakeholder meetings.
- In general, the feedback on the proposed changes was positive insofar as submitters felt the proposed changes were an improvement on the current list of nine decision criteria. Many respondents felt that the proposed decision-making matrix in association with the supporting information document provided greater clarity around how PHARMAC makes its funding decisions.
- The proposed decision-making matrix itself was supported by most respondents, though a number of submitters questioned how weightings would apply, and there was some confusion as to whether size of boxes corresponded with the importance of a given factor. Many respondents raised concern about how the factors for consideration could be applied to all decisions that PHARMAC makes; decisions in relation to people with rare conditions was highlighted by a number of respondents as not fitting into this framework. A small number of submitters also noted differences in relation to hospital medical devices.
- Most feedback was very positive about the inclusion of the Treaty of Waitangi in PHARMAC's decision making framework, but there were split views as to whether this should be an identified factor for consideration as well as part of the broader framework, or only the latter. Feedback was also generally very positive around the broadening of PHARMAC's consideration of populations with disparities. However, some submitters objected to people with rare conditions being excluded from the definition of 'population.'
- Respondents were generally very supportive of the proposed Supporting Information document, with feedback demonstrating that respondents felt the additional explanation was useful and necessary. Some submitters felt this level of detail should be formally included within PHARMAC's Operating Policies and Procedures (OPP).
- A number of respondents felt community values had not been adequately addressed in the proposed changes. Some submitters also felt that non-health outcomes such as broader socioeconomic impacts and environmental impact should be incorporated.

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## Introduction

This is a summary of submissions from stakeholders who responded to PHARMAC's consultation document *PHARMAC's Decision Criteria: Proposal for Change* via either direct submission to PHARMAC, attendance at a half-day Consultation Event, and/or through meetings held with PHARMAC staff. This is the second, and final, stage of consultation on PHARMAC's Decision Criteria.

The Decision Criteria review is part of a wider review of PHARMAC's Operating Policies and Procedures (OPP). We currently use the nine criteria as listed in Appendix 1, to make decisions about proposed amendments to the Pharmaceutical Schedule and decisions outside the Schedule relating to pharmaceutical treatments for named patients. Where PHARMAC makes decisions that do not involve amendments to the Schedule or named patients (for example, decisions relating to the promotion of the responsible use of medicine), we try to use these criteria, to the extent that they can be applied to those decisions.

The first round of consultation was held May-August 2013. In this round of consultation we received 139 written submissions, and ran a series of 12 community forums around New Zealand. Feedback received through this round of consultation informed the development of a proposal for change that was released for public consultation in March 2014.

PHARMAC thanks all those who have taken the time to be involved in this review of the Decision Criteria. A final announcement of the changes is expected in 2014.

## Methodology

PHARMAC launched this consultation process on 18 February 2014, making an announcement by media release and providing access to the consultation document on the PHARMAC website. All those who had registered an interest were informed of this via an email. This included (but was not limited to) those who had submitted an application in the first round of consultation and/or attended one of the community forums.

All stakeholders were encouraged to submit their feedback through to the OPP mailbox by the closing date of 21 April 2014, or contact PHARMAC to arrange a time to discuss feedback in person. One industry group contacted PHARMAC to organise a meeting. Feedback was also gathered through some of PHARMAC's regular stakeholder meetings.

PHARMAC also held a half-day Consultation Event on Wednesday 15 April, which was open to the general public, with the Decision Criteria being one of the three agenda items. Eighty people attended this event. In addition, PHARMAC also facilitated a meeting with a group of Māori stakeholders to discuss the Decision Criteria. Given we did not receive any submissions specifically from Māori representative groups in the first round, we felt this necessary to ensure a Māori perspective was heard.

PHARMAC received 49 written submissions, with a handful of these being received after the closing date. The table below provides a breakdown of the submitter-type.

<b>Submitter type</b>	<b>No. of submissions</b>
Consumer groups	9
DHBs and clinicians	3
Industry representatives (including Pharmaceutical and Medical Device organisations and industry groups)	8
Professional Associations	6
Public	18
State Sector Agency	5

## Structure of the Summary

*PHARMAC's Decision Criteria: Proposal for Change* consultation document included 21 consultation questions intended to prompt submitters' thinking on the proposed changes. There was some overlap in some of the questions, and in submitters' responses to the questions. In addition some submitters provided general commentary rather than responding explicitly to the questions posed. General feedback was also received through the Consultation Event and through the other meetings held with stakeholders. For this reason, this summary of submissions will group the consultation questions into key areas where appropriate, in order to incorporate all the feedback received. The key areas and consultation questions are outlined below:

<b>Key area</b>	<b>Consultation questions</b>
Overall approach	<ol style="list-style-type: none"> <li>1. How helpful is a high-level summary in better explaining what PHARMAC takes into account?</li> </ol>
Proposed decision-making Matrix	<ol style="list-style-type: none"> <li>2. How would the presentation of a decision-making matrix provide clarity over what PHARMAC considers when it makes a funding decision?</li> <li>3. Should the decision-making matrix be applied to all PHARMAC's decisions including Schedule, Named Patients and implementation decisions? Why or why not?</li> <li>4. Are there other dimensions that you would include? Are there any dimensions that you would leave out? Why?</li> <li>5. What alternatives to the proposed decision-making matrix could PHARMAC use for presenting what it takes into consideration?</li> </ol>
Factors for consideration	<ol style="list-style-type: none"> <li>2. How well would the proposed terminology 'factors for consideration' reflect how PHARMAC does or should think about its funding decisions? What other options can you suggest for describing these?</li> <li>6. What factors for consideration could be omitted or what further ones could be included to inform PHARMAC's decisions?</li> <li>13. What is your view on the proposed rewording of the factors for consideration?</li> </ol>

	14. Which factors, if any, are unclear or confusing?
Broader framework	8. How useful is it to frame the factors for consideration within the broader operating environment that PHARMAC operates within? 9. What key strategic or legislative obligations would you omit or include? Why?
Health Disparities	10. What would be achieved by broadening the health disparity factor to include any population groups experiencing health disparities?
Treaty of Waitangi	11. How is the Treaty of Waitangi best reflected in the proposed framework? Why?
Removal of Criterion 9	12. What would be the impact of removing the current decision criterion 9 ("such other criteria as PHARMAC thinks fit")?
Supporting Information	15. How helpful would the inclusion of a supporting information document be? How would the draft document (appendix 5) provide more clarity and transparency?
Medical Devices	16. What are the pros and cons from using the same decision-making matrix decision criteria for medicines and medical devices? Why? 17. What additional considerations relevant to medical devices could be captured in the proposed decision-making matrix?
Community Values	18. Does the proposed approach reflect your views on 'community values'? Why or why not? 19. What aspects of your 'community values,' do you feel are not captured in this proposal?
Non-health outcomes	20. Is there other rationale that PHARMAC hasn't considered that could be employed to justify PHARMAC considering factors related to non-health outcomes? What is this?
Considering patients with rare disorders	21. How well does the proposed approach adequately address the considerations that are relevant to funding proposals for treatments for rare diseases? Why?

## Overall approach

### **General Comments**

A large number of submitters and stakeholders engaged through the Consultation Event and other meetings were supportive of the proposed changes insofar as they felt they were an improvement on the current nine decision criteria. The matrix and the supporting information were acknowledged as providing more clarity and transparency around how PHARMAC makes its funding decisions.

A key theme that emerged from submitters across all submitter-types was the application of weightings in PHARMAC's decision making. Some questioned whether the proposed matrix depicted what factors may be given more weighting, others questioned if weightings of factors for given decisions could be made more transparent. Overall it was unclear amongst these submitters if, how and when weightings were applied in PHARMAC's decision making. Attendees at the Consultation Event also questioned how weightings were applied, with some attendees recommending values be associated with some of the factors in order to enhance transparency and demonstrate which have more importance.

Five submitters (including a consumer group, industry representatives and a member of the public) felt that a review of the decision making criteria could not be undertaken separate to a review of the decision making process. Two of these submitters acknowledged that PHARMAC had addressed the scope of this review in the *Proposal for Change* document, but reiterated the need for review of the decision making process to be aligned. One industry representative noted, "Impact of change hinges as much on how [the proposed changes to the criteria] are implemented as what constitutes the criteria."

Related to process, four submitters representing industry and consumer groups, highlighted the need for stakeholder and patient input to be a critical part of PHARMAC's decision making. This was also raised at the Consultation Event with attendees wanting more patient and clinical representation in decision making, and for there also to be a clear process for patients to submit on funding decisions. The process around the communication of PHARMAC's decisions was also raised at another regular stakeholder meeting held by PHARMAC.

Three industry representatives also raised overall concern in their submissions about 'pharmaceuticals' not incorporating medical devices, and therefore questioned the applicability of the proposed model to Medical Devices.

A member of the public and an industry submitter included an attachment to their submission with proposed new criteria to replace the current decision criteria. Another industry representative attached a document outlining proposed definitions of the factors for consideration; this submission was supported by two other submitters.

Ten submissions from members of the public were sent in support of one submitter who raised concern about pharmaceuticals for rare and fatal conditions that are not currently

funded by PHARMAC, and outlined some key principles and additional criteria. This feedback is captured in the relevant sections below.

### ***High-level summary***

Most submitters who commented on the proposed high-level summary found it helpful and appropriate for the purpose of providing an overall ‘snap-shot’ (11 submitters). One professional association noted the particular importance of the summary for health literacy purposes. A submission from an industry representative suggested that the high-level summary could be better depicted through overlapping circles with PHARMAC’s goal being where the three areas intersect (i.e. “patient’s unmet health need, can be met with a suitable treatment, which has an acceptable impact on the health sector”).

Three industry submitters did not find the high-level summary useful, noting that it oversimplified what is taken into account and provided no additional clarity on how the factors are used. Two further submitters also noted that it should be acknowledged at this level the differences across medicines, medical devices, hospital medicines and vaccines.

## **Decision-making Matrix**

### ***General Comments***

Eleven submitters across all submitter-types, supported this proposed framework to replace the current nine decision criteria. Submitters felt it enabled more information to be conveyed, was clear and helpful, and one professional association felt it enhanced PHARMAC’s credibility. One submitter also supported having the patient first. Some attendees at the Consultation Event also shared this view.

Of the eleven submitters, seven felt it was broad enough to be applied to all decisions PHARMAC makes including Schedule listings, medical devices and named patients. Two submitters recommended that, although the same factors should apply, weighting of the factors should be different for different decisions, and moreover should also differ depending on the “evaluator.” One of these submitters provided the example of the Pharmacology and Therapeutics Advisory Committee (PTAC) considering different factors to those considered by the PHARMAC Board. Two submitters supported the matrix with the caveat that each factor should be supported with a well-defined explanation.

Three submitters explicitly commented that they disliked the proposed matrix, with a number of other submitters outlining changes or elements of the matrix that they felt needed improvement.

Two submitters preferred the list of criteria to the proposed model. One member of the public noted that a list is easier to understand, and provides a more natural way of analysing data. Another member of the public commented that the matrix further obscures the reasoning behind PHARMAC’s decision making rather than providing clarification. A state-



sector agency also questioned if the display of the factors in the format of the matrix achieved its goal in terms of demonstrating the inter-connectedness of the factors.

One industry representative questioned if some 'factors' were criteria and if others were merely considerations. Another submitter felt the matrix did not give due weight to the emphasis placed on cost-effectiveness and budget impact. Similarly, a consumer group also voiced concern that the matrix may encourage "inside the box" thinking, meaning some costs, benefits and needs that are not part of the matrix will be omitted.

Six submitters (including members of the public, industry representatives and a consumer group) disagreed that the proposed matrix could be applied to individual patients. These submitters believed the current proposal had been developed for large populations and doesn't enable consideration of the relevant individual variables. Two further submitters noted that there needed to be additional flexibility when considering complex issues particularly around rare conditions. One submitter recommended that single processes could be developed for different types of decisions or alternatively an 'anything else' category could be included to enable the different variables and unique circumstances to be incorporated. Some attendees at the Consultation Event also felt that the proposed matrix was sufficient for 'technologies' but not all situations, and therefore did not feel that the one model was able to be used for all decisions.

Some submitters (a professional association, member of the public and an industry representative) felt that the reference to the 'health sector' was too narrow and the matrix should be more encompassing of wider societal and environmental considerations. This would include for example "interdisciplinary care and whanau community," as recommended by one professional association. This view was also shared by some attendees at the Consultation Event, with clarification requested on what the health sector encompassed.

Some attendees at the Consultation Event felt the matrix was too broad, and raised uncertainty around how this new framework would translate into funding applications.

### ***Suitability dimension***

Seven submitters specifically discussed the suitability dimension in their submissions. Five of these submitters (including from DHBs and industry representatives) questioned the purpose of this dimension; i.e. what is covered in this dimension that is not included in the other dimensions? This point was also questioned at the Consultation Event. One industry representative recommended 'suitability' be replaced with another column which would include safety/efficacy, education and support.

Two submitters (both industry representatives) strongly supported the inclusion of the suitability dimension as long as there was opportunity for patient perspective to be included as part of the process.

### ***Additional dimensions***

A number of submitters recommended additional dimensions should be included into the matrix. These included:

- community values;
- timeframes (including making decisions in a timely manner);
- society;
- collaborative approach across Government;
- other;
- public and consumer views;
- acute or chronic use;
- compliance effect;
- equity and ethics; and
- public accessibility (i.e. efficiency of access).

### **Factors for consideration**

#### ***General Comments***

Nine submitters (including consumer groups, industry representatives, professional associations and state sector agencies) supported the change from criteria to factors for consideration. Submitters who discussed the change in terminology felt 'factors for consideration' is more meaningful and indicative of the process. Submitters felt that the detail in the factors themselves provided a useful intermediate level between principles and supporting information. The factors were also found to be easier to interpret, though one submitter felt a footnote with an explanation of the factors would be useful. One professional association felt that the factors provided greater clarity and explanation, particularly in association with the supporting information.

Four submitters preferred the terminology of 'criteria' (three industry representatives and a member of the public). One industry representative felt that 'factors for consideration' do not project a fair, transparent and accountable framework. Another industry representative felt that it was critical for each of the 'factors' to be met prior to making a decision and therefore they should be used and applied as 'criteria.'

A number of other submitters raised general concerns about the factors lacking clarity. Two submitters (member of the public and industry representative) found the factors too vague and felt they lacked transparency, with one of these submitters suggesting this diluted PHARMAC's accountability. One submitter also noted that the terminology around the matrix of "may be taken into account" and "where applicable" further reduces transparency. An industry submitter disputed PHARMAC's explanation that there is no simple calculation to apply the factors, noting that PHARMAC has the authority to decide which factors are essential and which are discretionary.

Some submitters, and some attendees of the Consultation Event questioned how PHARMAC takes more 'values-based' factors into account, with a number of submitters and attendees recommending more 'social benefits' be accounted for, as well as consideration of factors outside of health (these are noted more specifically in the list and the table below). One member of the public stated that the value and importance of human life is understated in PHARMAC's decision making. Another submitter felt that PHARMAC should be more explicit in considering the cost of not funding a medicine or medical device, particularly for vulnerable people. One submitter felt that public health considerations should take precedence over other factors (such as cost-effectiveness) when considering, for example, vaccines.

One submitter from a professional association felt that factors were too circumscribed, and didn't take into account longer-term outcomes, primary health care priorities and the ageing population. The same submitter also felt that there was too narrow of a focus on *treatments for patients*, which missed the opportunity to tailor to the unique population needs of Aotearoa.

### ***Additional factors for consideration***

Submitters and attendees at the Consultation Event recommended the inclusion of a number of additional factors for consideration, that they felt were not captured in the proposed matrix. These included:

- an avenue for stakeholder input;
- the 'cost of delay' in not funding a decision;
- inclusion of opportunity cost of funding (and not funding);
- urgency of availability;
- individuals' ability to be an active participant in society and to be self-reliant;
- clinical Safety; including both patient and clinician safety factors. A state sector agency recommended the following considerations in relation to clinical safety:
  - medicines with multiple preparations
  - look-alike medicine labelling and packaging
  - sound-alike medicine names
  - implications of changing brand/ formulations for the prescriber/ dispenser/ administrator (need for additional patient education re dose alteration etc).
- rarity;
- impact on the family and whanau;
- costs now relative to future costs;
- reducing the risk of other complications;
- social burden; and
- costs broadened to include ethical, human and social costs.

A number of additional considerations were also noted in relation to medical devices. To avoid repetition these will be discussed in the medical devices section on pages 14-16.

### **Feedback on specific factors for consideration**

A number of submitters also commented on the proposed wording of most of the factors for consideration. This feedback has been collated into the table below.

<i>Factor for consideration</i>	<i>Feedback</i>
Health need of the patient population under consideration relative to all eligible people within NZ	<ul style="list-style-type: none"> <li>• Not just about illness should also include, for example, “well women’s reproductive health needs such as contraception”;</li> <li>• health need of children should be identified and prioritised;</li> <li>• health need of those with rare and fatal conditions should be prioritised;</li> <li>• should include ‘unmet’ health need;</li> <li>• ‘relative to all eligible people within NZ’ excludes those with rare disorders given small population number.</li> </ul>
The principles of The Treaty of Waitangi	<i>Discussed in more detail on page 15-16</i>
The impact on the health outcomes of population groups experiencing disparity including Māori and Pacific	<i>Discussed in more detail on page 14-15</i>
The availability and suitability of existing medicines, medical devices and treatments for the clinical use under consideration	<ul style="list-style-type: none"> <li>• Should include cost-effectiveness and value of existing medical devices;</li> <li>• include ‘currently funded’ after availability and suitability.</li> </ul>
Supporting Government health priorities	<ul style="list-style-type: none"> <li>• Should be removed.</li> </ul>
Clinical benefits and risks of the medicine or medical device to the patient and health outcomes	<ul style="list-style-type: none"> <li>• Should not enable more experimentation with medicines in particular ;</li> <li>• supporting information does not acknowledge patient safety as part of clinical benefits and risks;</li> <li>• acknowledge evidence is less readily available for In-Vitro Diagnostic (IVD) medical devices;</li> <li>• supplier characteristics (as discussed in supporting information) needs to expand beyond stock and supply</li> <li>• Include ‘both to patient outcomes and to the health sector’;</li> <li>• should include ‘natural history studies’ where people have been treated with success;</li> <li>• should be explicit in wording that PHARMAC is considering health effects on members of society as well (such as in the case of vaccines);</li> <li>• ignores impact on family, social and wider implications on community;</li> <li>• replace ‘health outcomes’ with ‘community’ or ‘society’;</li> <li>• more detail required as to what clinical benefits and risks are included.</li> </ul>

Benefits and risks to the health sector of the medicine or medical device	<ul style="list-style-type: none"> <li>• Include benefits and risks to communities or society.</li> </ul>
Out of pocket costs to the patient using the medicine or medical device	<ul style="list-style-type: none"> <li>• Need to ensure decisions negate or minimise costs so access to treatments is enhanced;</li> <li>• should include cost of not funding the treatment as well;</li> <li>• should be 'out of pocket costs' to 'health service users';</li> <li>• should include costs to the patient and the family, now and in the future;</li> <li>• replace colloquial 'out of pocket' with 'personal'.</li> </ul>
Cost of the medicine or medical device	<ul style="list-style-type: none"> <li>• Too narrow - should be cost-effectiveness in relation to other alternative treatments;</li> <li>• consider 'cost-offsets';</li> <li>• should refer to cost-effectiveness or some other measure of cost relative to therapeutic value;</li> <li>• should include "...to the patient and the family, now and into the future".</li> </ul>
Flow-on costs of the medicine or medical device to the rest of the health sector	<ul style="list-style-type: none"> <li>• Should also consider flow on benefits and savings to the rest of the sector (eg, investment in pharmacotherapy may result in reduced Emergency Department attendances or hospital admission);</li> <li>• consider costs to private bodies as well, such as charities operating in the sector (eg, if a funding change requires additional support from a charity to help people learn about a change in device);</li> <li>• include costs and risks associated with implementation;</li> <li>• future costs of a patient and the total cost of the health of that patient, and if spending money on treatment early can decrease the need for increased care in the future;</li> <li>• should include the cost of not funding.</li> </ul>
Suitability of the medicine or medical device to the patient	<ul style="list-style-type: none"> <li>• Should include patient testing for usability;</li> <li>• parents'/caregivers' views on suitability should be considered for vaccines in particular as often administered to young children;</li> <li>• broad term so should include considerations such as allowing for individualised patient side effects to treatments.</li> </ul>

### **Broader framework**

Almost all submitters who discussed the proposal to include the broader framework that PHARMAC operates within were supportive of the idea and the proposed model (13 submitters across all submitter-types). One professional association recommended a web-based reference to the overarching legislative and strategic documentation would be particularly useful. Another professional association noted that 'rest of OPPs' is possibly a

bit opaque. Two industry representatives also suggested the broader framework should be framed in the context of government priorities and initiatives.

Some industry representatives raised concern about the lack of recognition of medical devices, both in reference to the terminology of 'pharmaceutical' and the lack of strategic documentation incorporating medical devices. One industry group questioned if a strategy equivalent to the Medicines New Zealand Strategy existed for medical devices.

One consumer group criticised the lack of acknowledgement on how the legal framework and guiding documents influenced decision making or how they would be incorporated into PHARMAC's decision making. An industry representative also commented that on the basis of the proposed framework they would expect a right to appeal.

Attendees at the Consultation Event particularly emphasised PHARMAC's responsibilities under the Human Rights Act, and felt that these were not adequately captured in the proposed framework.

Submitters also recommended the following legislative or other obligations be included:

- Health and Disability Commissioner Act;
- United Nations Convention on the Rights of Persons with Disabilities;
- Article 25 in the Universal Declaration of Human Rights;
- WHO Right to Health;
- Commerce Act;
- New Zealand Medicines Act 1981;
- 'international attempts to eradicate vaccine preventable diseases as recommended by WHO';
- International Covenant of Economic, Social and Cultural Rights;
- gazette notes expanding PHARMAC's function; and
- Memorandum of Understanding with DHBs.

## **Health disparity**

Six submitters (including public, industry representatives and professional organisations) supported the proposed broadening of this criterion to include other population groups experiencing health disparity. One industry submitter noted this was a meaningful attempt to respond to the established need in defined populations. Another submitter from a professional association also noted that broadening this factor would enable the collection of comprehensive and specific data enabling fairer and more cost-effective targeting.

Conversely, seven submitters and some attendees of the Consultation Event were strongly opposed to the broadening of this factor, specifically in relation to not including 'people with rare conditions' as a population group. Submitters of this view (including members of the public, industry, consumer groups and a state-sector agency), felt that the health system as a whole caused this group within the population to be at a significant disadvantage, one member of the public noted that PHARMAC's decision making process has led to this health

disparity. A consumer group also argued that this factor was not broadening the previous criterion but rather narrowing by determining disparity by geographical, demographic or socioeconomic characteristics. This submitter noted that 'rare conditions' would always fail at the "cost/benefit hurdle" when stacked up against the health needs of other New Zealanders.

Other submitters also questioned how 'disparity' would be defined and how those groups will be identified. One industry submitter noted a challenge for PHARMAC will be how to determine and measure health disparity and appropriately 'weigh' the health needs of minority groups. Relatedly another consumer group submitter noted that 'health outcomes' can not be considered in isolation, and wider social, community and economic effects must also be taken into account.

A professional association commented that disparities should not just be considered in new medicines but also in medicines already funded, in relation to access. They note that there is no point in considering patients with disparity if these patients are already not accessing medicines.

Three submitters (two professional associations and a member of the public) questioned the explicit reference to Māori and Pacific peoples. One submitter noted that though Māori and Pacific peoples are disproportionately affected in terms of, for example, poorer economic, social and educational statistics, this is not caused by their ethnicity. Another submitter supported this, and objected to the stereotyping of Māori and Pacific peoples having health disparity. This submitter also noted that obligations under the Treaty of Waitangi should not be confused with addressing health disparities, historic or otherwise. This view was also supported by a meeting of Māori stakeholders that were brought together to discuss the proposed changes to the Decision Criteria. This group additionally felt the needs of Māori should be considered separately to other population groups facing disparity given they are the tangata whenua.

## **Treaty of Waitangi**

Almost all submitters who commented on the explicit inclusion of the Treaty of Waitangi ("the Treaty") in PHARMAC's decision making framework supported this proposal. This view was also supported by attendees at the Consultation Event and attendees of the Māori Stakeholder meeting. Six submitters supported the inclusion of the Treaty as both a factor for consideration and in the wider framework. One industry submitter noted the importance of the Treaty in the consideration of medical devices as well as medicines.

However, five submitters questioned why the Treaty was included in both the broader framework and in the matrix; these submitters supported the Treaty being at the level of the legal and strategic framework so it is incorporated into all of PHARMAC's work. Similarly, three submitters (a state-sector agency, a professional association and an industry representative), and attendees at the Māori Stakeholder meeting felt that the principles of the Treaty should be considered under each of the dimensions of the matrix, not just 'need.'

One submitter from a professional association recommended it sit alongside the statutory objective in terms of being a touch point for every decision.

Attendees at the Stakeholder Event felt it was also important to include PHARMAC's Te Whaioranga Strategy. This view was supported by attendees of the Māori stakeholder meeting. Attendees of this meeting also felt it was important for PHARMAC to reference Te Tiriti (instead of The Treaty) and also that PHARMAC staff are informed about applying the principles of Te Tiriti.

One submitter felt that Māori should not be prioritised in PHARMAC's decision making framework.

### **Removal of criterion 9 (such other criteria as PHARMAC sees fit)**

Of the 18 submitters who responded to the question regarding the removal of this criterion, 14 submitters were in support. Two submitters supported its removal, with the caveat that it is made clear that any other relevant factors will be consulted on, and in addition the factors themselves are fully comprehensive. One industry submitter also supported removal so long as the current factors could be flexible enough to take into account, for example, "the potential for leap-frog technological developments."

Four submitters opposed the proposal to remove this criterion (two industry submitters, a member of the public and a professional association). These submitters opposed its removal on the basis that the proposed matrix does not enable sufficient flexibility to consider, for example, exceptional circumstances, PHARMAC's expanding remit, and the differences specific to vaccines.

### **Supporting information**

Submitters in general were really supportive of the proposed inclusion of a supporting information document, with 11 submitters agreeing that it was a necessary addition and provided more clarity and transparency. Three submitters noted the importance of regularly reviewing and updating this document, with an industry submitter noting that stakeholder input should also be considered. An industry submitter requested more real-life examples of successful and unsuccessful funding decisions (acknowledging limitations around confidentiality).

One industry representative stated that the document provided no additional clarity given it was so strongly couched in disclaimers. The submitter also questioned the rationale for presenting factors that are not taken into account.

Three industry representatives and one consumer group felt that the supporting information should be included as part of the OPP document. One industry submitter felt it was critical that definitions be included in the OPP to clarify the precise meaning attributed to each of the factors for consideration. All of these submitters felt unsure of the value of the OPP without this explanatory information included and felt without it, it would open the factors up to a



wide range of interpretation. These submitters requested the material to be part of the OPP be formally consulted on again, if the supporting information (or definitions) were to be included.

## Hospital Medical Devices

Of the submitters that provided feedback specifically in relation to medical devices, views were split as to whether the same framework should be applied to medical devices.

Seven submitters supported using the same framework and factors for consideration for medical devices, including consumer groups, an industry representative and professional associations. Of these submitters, three acknowledged that differences exist and noted the importance of ensuring the supporting information captured the nuances. Two submitters noted additional costs associated with medical devices would need to be accounted for within the proposed framework. A professional association recommended PHARMAC consider a 'Multi-Criteria Decision Analysis' approach whereby different weights are given to each criterion to rank importance. The industry representative noted that as pharmaceutical companies focus on personalised health care, it makes more sense to consider, for example, pharmaceuticals and their companion diagnostic tests, under the same matrix.

Five submitters (including industry representatives and members of the public) felt that there should be a more specific or appropriate set of criteria (or decision making framework) for medical devices, with two of the industry representatives asserting that there would be no advantages in having the same criteria. These submitters felt that the current proposed matrix is too simplistic in its consideration of medical devices. One submitter felt that though Quality-Adjusted Life Years (QALYs) and Cost-Utility Analysis (CUA) are applicable to the analysis of medicines, these are unable to be derived for devices given the challenge of conducting randomised control trials and comparing evidence across products.

Other key differences that submitters noted in respect of medical devices included:

- different life-cycles compared to medicines;
- different evidence base;
- additional training and expertise required;
- consumables;
- deployment times are different;
- contracts for medical devices tend to be longer;
- IVD devices in particular are less substitutable than pharmaceuticals;
- weightings of the factors need to be different for devices;
- need to recognise categories and clusters of devices;
- decisions need to be made in a relatively short time-frame to ensure technology is up to date;
- devices cannot be assessed in isolation (part of a complex set of technology);
- custom-made devices may have relevance for only one patient;
- transparency in relation to 'bundling'; and
- may have instant life-saving benefits.

Two submitters from state sector agencies pressed the importance of patient safety and quality and said that this should be at the forefront of any decision relating to medical

devices. One of these submitters noted that as there is much less data available about the effectiveness and safety of medical devices, PHARMAC needs to be explicit about the consideration of patient safety.

A number of industry submitters recommended some additional factors for consideration that should be included specifically for medical devices, or in order to encompass differences for medical devices. These included:

- national health impacts and priorities;
- additional budgetary requirements;
- local suppliers;
- product quality;
- usability for patients and staff;
- maintenance and/or ongoing consumable costs;
- evolution of devices over time;
- ability of the device to address workforce issues;
- trends in healthcare outside of New Zealand;
- improvement of staff safety;
- an acute or chronic dimension;
- compliance effect;
- protection of intellectual property;
- cost of change;
- opportunity cost of not funding;
- whether device is already in use in either private or public settings;
- contribution to economic growth that innovation can bring;
- whether whole or significant components are produced in New Zealand;
- often limited evidence available particularly for new devices;
- device may have instant lifesaving benefits and so delay in funding may mean loss of life;
- disinvestment of old technology; and
- custom-made devices by definition only have relevance for a specific patient.

## **Community values**

In PHARMAC's consultation document, there was a discussion around why community values were not included in the proposed changes to the Decision Criteria. Five submitters, including industry representatives, a consumer group and a professional association agreed with PHARMAC's rationale, particularly referencing that there is no homogenous definition of 'community values.' The professional association felt that the changes permitted some community values through addressing health disparities within communities.

Eight submitters did not agree with the rationale that PHARMAC had provided for not explicitly incorporating community values as a factor for consideration. One industry submitter felt that it was contradictory that PHARMAC practices an "unstated preference for a utilitarian framework" yet is unable to include community values into its decision making. A member of the public felt that some community values were widespread, such as the

importance of saving human life. This submitter felt a consultation on what community values to consider would have been more constructive. Relatedly, another submitter felt community values should be included as a factor, and that some form of assessment would be possible. An industry representative noted research carried out by Otago University that shows meaningful conclusions can be drawn from a survey into community preferences.

A number of submitters, including consumer groups, industry and a professional association felt aspects of their community values were not captured in PHARMAC's proposal. These included:

- full participation in society;
- full participation for the most vulnerable in society;
- pharmaceutical savings being reinvested back into pharmaceuticals/ treatments;
- patients, families and their health care providers forming the centre of decision making and prioritisation;
- equity of health outcomes (including universality and access to services for most vulnerable);
- opportunity costs in terms of health outcomes;
- sustainability;
- rule of rescue;
- human rights – right to life and right to health;
- legal considerations such as the use of a vaccine in the Schedule different from that recommended by the manufacturer;
- clinical choice; and
- at a minimum to do no harm to the community (specifically in reference to medical devices).

## **Other rationale to justify non-health outcomes**

A number of submitters provided rationale to justify PHARMAC including non-health outcomes in their decision making.

The inclusion of relevant socioeconomic factors was recommended by some submitters. A member of the public and an industry representative noted the importance of retaining employment and not needing assistance with daily living. A professional association recommended PHARMAC use QALYs in order to include wider societal benefits.

Three industry submitters felt innovation in all health technologies should be a relevant non health outcome that PHARMAC should take into account. One of these submitters also felt the impact of decisions on supplier viability to operate in New Zealand should be taken into account, as this may ultimately lead to decreased choice for New Zealanders.

An industry submitter and a professional association still felt environmental impact and sustainability were important factors, although one of these submitters noted a clear framework would be required. Relatedly, another professional association felt consideration of medicines being available in smaller pack sizes or minimal packaging in order to minimise

waste should be considered. This submitter also felt that PHARMAC or manufacturers should fund the collection and destruction of returned or unwanted cytotoxics.

### **Addressing consideration of patients with rare conditions**

PHARMAC asked how the proposed approach addresses considerations that are relevant to funding proposals for treatments for rare diseases. Two submitters (a consumer group and a professional association) agreed the approach presented by PHARMAC was reasonable, with the professional association noting that the use of sound clinical evidence, cost-benefit analysis and weighing up all factors will ensure transparent decisions are made.

Eleven submitters (including consumer groups, industry, professional associations and members of the public) disagreed that the current approach adequately meets the needs of patients with rare conditions. This view was also supported by some attendees of the Consultation Event.

A consumer group felt that contrary to what PHARMAC had said in the consultation document, the circumstances around the development of treatment for a rare condition make these treatments at a disadvantage in PHARMAC's current processes, due to factors such as high research and development costs and low patient numbers for trials. A member of the public also reflected this point noting that despite PHARMAC acknowledging that lack of evidence is compensated for during evaluation, PHARMAC will then decline an application on this basis. Two submitters also raised concern that the current proposal does not address the lack of access to treatments for people with rare disorders.

Other submitters felt that rare disorders have a number of nuances that are not reflected in the factors for consideration, with most submitters highlighting the economic realities of developing an orphan treatment compared to 'blockbuster drugs.' A member of the public and a consumer group commented that it is not possible for people with rare disorders to fit into the same 'silos' as those with common health conditions. Six submitters (a consumer group, an industry representative, professional associations and members of the public), and some attendees at the Consultation Event felt there must be a separate and dedicated fund for considering treatments for rare disorders.

A number of submitters reflected on the discussion document that PHARMAC had recently released on the contestable fund for rare disorder medicines. Two submitters (industry and professional association) felt that the release of this document may address some of the concerns raised around funding for medicines for rare disorders. A consumer group and a member of the public raised confusion around what they felt was a contradiction in the discussion document and the proposal for change around how 'population' was defined.

Some attendees at the Consultation Event felt rare conditions should be referenced in the matrix, and some felt the framework does not fit this group of people.

## Appendix 1

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 amendments to the Schedule. Where PHARMAC makes decisions that do not involve amendments to the Schedule (for example, decisions relating to PHARMAC's demand side activities), it endeavours to use these criteria, to the extent that they can be applied to those decisions. The criteria for decisions about proposed amendments to the Schedule are:

- a) The health needs of all eligible<sup>1</sup> people within New Zealand;
- b) The particular health needs of Māori and Pacific peoples;
- c) The availability and suitability of existing medicines, therapeutic medical devices and related products and related things;
- d) The clinical benefits and risks of pharmaceuticals;
- e) 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 pharmaceutical 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 things fit.

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<sup>1</sup> As defined by the Government's then current rules of eligibility.