PHARMAC's Decision Criteria

Proposal for Change

February 2014

newzealand.govt.nz

PHARMAC
Pharmaceutical Management Agency

Contents of consultation document

1. Context and review process

1.1 Origins and aims

PHARMAC is currently reviewing its Operating Policies and Procedures (OPPs). These are PHARMAC's framework for how we carry out our statutory role of deciding, on behalf of District Health Boards, which pharmaceuticals and related products are subsidised for use in the community and in public hospitals. They provide guidance to the people and groups we work with, about what to expect when working with us, and they guide us internally as we consider funding proposals and policy changes. We last reviewed our OPPs in 2005, and since then PHARMAC's role has expanded. In particular, the decision criteria were developed before we expanded our role into hospital medicines and medical devices.

PHARMAC's decision criteria form part of our Operating Policies and Procedures (OPPs). We currently use the decision criteria, as they are set out below, to make decisions about proposed amendments to the Pharmaceutical Schedule and decisions outside the Schedule relating to 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 OPPs currently include the following section regarding the decision criteria:

> 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[1] people within New Zealand;
>
> b) the particular health needs of Maori and Pacific peoples;
>
> c) the availability and suitability of existing medicines, therapeutic medical devices and related products and related things;
>
> d) the 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;

[1] As defined by the Government's then current rules of eligibility.

h) the Government's priorities for health funding, as set out in any objectives notified by the Crown to PHARMAC, or in PHARMAC's Funding Agreement, or elsewhere; and

i) such other criteria as PHARMAC thinks fit. PHARMAC will carry out appropriate consultation when it intends to take any such "other criteria" into account.

1.2 Process to date

The formal review of our OPPs began with a discussion at the PHARMAC Forum on 20 February 2012. In April 2012, we released a discussion document seeking feedback from the public on what should be included in the OPPs. In response to the submissions we received, in early December 2012, we released notification of:

- The list of topics to be included in the revised OPPs
- Our intention to re-develop the OPPs as a web-based guide
- Our intention to begin a rolling review of the substantive content of the OPP topics (and thus PHARMAC practice), starting with a review of our nine decision criteria

The first round of consultation on the decision criteria began in May 2013 with the release of a consultation document. Over the following three month consultation period we held a series of 12 community forums at different venues around New Zealand, with over 300 people attending. We really valued the opportunity to engage directly with the public and our stakeholders, and were very pleased with the constructive feedback we received at these forums. In addition, we received 139 written submissions from government organisations, consumer and community groups, clinicians, medical and pharmaceutical groups and individuals, and held meetings on request with five industry and government groups. We thank all of those who took the time and effort to engage with us in this first round of consultation, and we look forward to receiving your further feedback on the proposed changes.

Following our review of all the feedback received over this consultation period a Summary of Submissions was published on the PHARMAC website in December 2013, reflecting back what we heard during this first round of consultation.

Key themes that emerged from this first round of consultation included the following:

- Most submitters considered the criteria should be amended to varying degrees. Although many submitters noted the criteria largely work well, some criticised PHARMAC for how the criteria are applied in terms of the consistency and transparency of funding decisions.
- Of those submitters who shared their personal experiences with the decision criteria, many commented that they felt the fiscal impact of the funding decision outweighed the consideration of other criteria. Others acknowledged the need for criteria to be flexible, especially in consideration of rare or complex conditions; however, some also felt that a degree of specificity was required.
- Submitter opinion was split in relation to the appropriateness of the current criteria for medical devices. It was widely felt that devices are more complex than pharmaceuticals. Some submitters felt amendment to the criteria would be required to encompass devices; others felt common criteria would be possible.
- Many submitters made recommendations about having an overarching set of principles to preface the criteria. Many submitters considered that the current criteria do not reflect fairness or community values.

In addition to the decision criteria review, PHARMAC has also been undertaking consultation on our work to establish a framework for the future management of hospital medical devices. The outcome of this work will also form part of PHARMAC's OPPs. Feedback received through the various medical devices' consultations that is relevant to the decision criteria review (and vice versa) has also been taken into consideration in the development of this proposal for change. The next consultation on the proposal for how the PHARMAC model will be applied to medical devices is intended to be released sometime in April. However, as this decision criteria proposal reflects how medical devices may be considered within the proposed decision-making matrix, this serves as a part of PHARMAC's proposal for applying the PHARMAC model to medical devices.

1.3 Seeking your feedback

This document outlines our proposed changes to the decision criteria based on what we have heard during the first round of consultation. Included throughout are consultation questions to prompt your thinking and feedback, and these questions have been collated in Appendix 1.

To the extent possible, we have described where the proposed changes reflect submission feedback, but we have also included a table in Appendix 2 that summarises all of our responses to the feedback we received, including those suggestions that have not been included in the proposed changes. In the initial consultation many submitters commented on the application of the decision criteria. The application of the criteria is more related to process than the decision criteria themselves, and therefore is beyond the scope of this review; however this feedback will be taken into consideration and will contribute to upcoming reviews of other aspects of PHARMAC's OPPs.

1.4 Submitting your response

Comments can be submitted via email, fax or letter by 5pm Monday 21 April 2014 to:

Rebecca Keat
PHARMAC
PO Box 10-254
Wellington 6143

Email: opp@pharmac.govt.nz
Fax: (04) 460 4995

We also invite interested people or groups to meet with PHARMAC staff to present their views in response to this consultation. Please contact Rebecca Keat at opp@pharmac.govt.nz by Friday, 21 March 2014 if you would like to arrange a time to meet with us. If a range of groups are interested in meeting we may organise larger group meetings.

Information requested under the Official Information Act

Feedback we receive is subject to the Official Information Act 1982 (OIA) and we will consider any request to have information withheld in accordance with our obligations under the OIA. Anyone providing feedback, whether on their own account or on behalf of an organisation, and whether in a personal or professional capacity, should be aware that the content of their feedback and their identity may need to be disclosed in response to an OIA request.

We are not able to treat any part of your feedback as confidential unless you specifically request that we do, and then only to the extent permissible under the OIA and other relevant laws and requirements. If you would like us to withhold any commercially sensitive, confidential proprietary, or personal information included in your submission, please clearly state this in your submission and identify the relevant sections of your submission that you would like withheld. PHARMAC will give due consideration to any such request.

1.5 Next Steps

After the consultation period closes, we will consider all of the submissions we receive, and will then provide a final proposal for change to the PHARMAC Board. We will be able to update you on the timeframes for implementing any changes following approval from the Board, and we will also release a summary of submissions.

Some people are interested in participating in the review of other areas of our OPPs. As the decision criteria are a central part of our OPPs, any changes that might be made to the decision criteria will need to be taken into account when we review other sections of our OPPs. We will announce in due course the next section to the OPP to be reviewed, following completion of this review.

2. Proposal for change

Some of the feedback we received during our first round of consultation indicated that there is some uncertainty regarding what PHARMAC actually takes into account when we consider a funding decision. Some feedback also indicated that it is not clear to external parties where the decision criteria sit within PHARMAC's wider operating environment, and how the criteria serve to achieve our statutory objective.

With these issues in mind, the proposed changes reframe our current decision criteria in a way that is intended to better reflect how PHARMAC considers whether decisions or proposals meet our statutory objective of securing for eligible people in need of pharmaceuticals the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the funding provided. The changes are intended to provide more transparency and clarity about our consideration of funding decisions.

We also acknowledge that the criteria were developed prior to PHARMAC expanding its role into new areas such as hospital medicines, vaccines and medical devices. We propose to make changes to reflect these new areas of PHARMAC's operation.

The key changes, discussed in further detail below, are intended to inform all decisions about proposed amendments to the Schedule and decisions outside the Schedule relating to treatments for named patients. Where PHARMAC makes decisions that do not involve amendments to the Schedule (for example decisions relating to the promotion of the responsible use of pharmaceuticals), we propose to apply the proposed approach, to the extent that it is relevant to those decisions.

2.1 Summary of what PHARMAC thinks about when it makes a decision

To respond to submitters concerns about the complexity of the current decision criteria, we propose to provide a high level summary in plain language of what PHARMAC thinks about when we make our decisions. This could be useful in demonstrating the range of factors that PHARMAC considers, however we also recognise that many submitters require greater levels of detail in order to more fully understand what PHARMAC takes into account. The factors we take into account are outlined in more detail in section 2.3 below.

PHARMAC thinks about the following when it makes its decisions:

- The patient's health need, the cost to them, how suitable the treatment is and how much they would benefit from it.
- The benefits, risks, and costs of the medicine or medical device, and whether there are any alternatives.
- How this would support achieving the Government's health priorities, and whether there are any benefits or extra costs to the wider health sector.

1. How helpful is a high-level summary in better explaining what PHARMAC takes into account?

2.2 Application of the factors PHARMAC considers when making funding decisions

Through our consultation feedback it became apparent that for some submitters the terminology of 'criteria' implies that PHARMAC may use a mandatory 'tick box' approach when applying the decision criteria i.e. that each funding decision is required to 'meet' each individual criterion. However, that is not the case. Whether or not a decision or proposal helps PHARMAC to achieve its statutory objective is what PHARMAC is legislatively bound to consider when it makes decisions.

There are a range of relevant factors that assist us to determine whether that is the case. PHARMAC needs to use its judgement to consider all of the relevant factors implicit in the current criteria and to consider each funding application against all of the other possible funding options. This flexibility in our decision making process was supported by a large number of submitters, who felt that it was important that PHARMAC was able to consider all of the relevant considerations, particularly in complex or unusual cases.

We propose outlining these relevant 'factors for consideration' to better reflect our actual decision-making process, while confirming that PHARMAC's statutory objective remains the touchstone for every funding decision that PHARMAC makes. The proposed factors are not an exhaustive list of what may be taken into account in a given decision and moreover not all the factors will always be relevant, as is the case with the current criteria.

The current decision criteria are applied by PHARMAC at several stages of the decision-making process and for a range of different types of funding decisions. As a result all current criteria are

not applicable to every decision. This is outlined in section 2.2 of our current OPP document as a preamble to the criteria, but we acknowledge that this could be made more explicit.

In addition, PHARMAC makes a range of decisions including, for example, Pharmaceutical Schedule listing decisions (including Tender decisions) and Named Patient Pharmaceutical Assessment (NPPA) decisions. Considerations for each of these different types of decisions are different, and therefore individual factors may have more or less bearing on the final decision. For example, for an Unusual Clinical Circumstances (UCC) NPPA decision, current criterion 2 (the particular health needs of Māori and Pacific Peoples) may have little or no relevance as the NPPA application is for an individual and therefore the health need of particular population groups is not a relevant consideration.

The 'factors for consideration' will help inform our consideration of whether a decision is consistent with PHARMAC's statutory objective, including determining what "best" means in the context of best health outcomes. The wording of the proposed factors for consideration is discussed further in section 2.6.

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?

2.3 Provide a decision-making matrix

In response to feedback we received during the consultation on our current approach, and a desire to better reflect how the proposed factors for consideration would be used, we propose to present this information within a decision-making matrix, as shown in the diagram on the following page.

As part of the matrix we propose to frame the factors in terms of various dimensions, including:

- benefits, suitability, costs and need; and
- how each these dimensions relate to the patient, the treatment, and the wider health sector (including clinicians and health professionals).

The advantages of framing the factors for consideration in this way include the following:

- It would further support PHARMAC's ability to consistently consider the impact of possible decisions at the patient, treatment and health sector levels.
- It would help demonstrate the inter-connectedness of all of the various factors and the need to use judgement to consider these impacts against each other.
- It would assist our stakeholders to identify the particular dimension that is of most interest to them, and how it relates to the other dimensions.
- It would help demonstrate the relationship between the factors for consideration and PHARMAC's statutory objective.

Proposed decision making matrix

	Need	Benefits	Costs	Suitability
Patient	The health need of the patient population under consideration relative to all eligible people within New Zealand	Clinical benefits and risks of the medicine or medical device to the patient and health outcomes	Out of pocket costs to the patient using the medicine or medical device	Suitability of the medicine or medical device to the patient
	The Principles of the Treaty of Waitangi			
	The impact on the health outcomes of population groups experiencing health disparities including Māori and Pacific peoples			
Treatment	The availability and suitability of existing medicines, medical devices and treatments for the clinical use under consideration		Cost of the medicine or medical device	
Health Sector	Supporting Government health priorities	Benefits and risks to the health sector of the medicine or medical device	Flow-on costs of the medicine or medical device to the rest of the health sector	Suitability of the medicine or medical device to the health sector

Statutory objective:

Does the proposal or decision help PHARMAC to secure for eligible people in need of pharmaceuticals the best health outcomes that are reasonable achievable from pharmaceutical treatment and from within the amount of funding provided?

3. How would the presentation of a decision-making matrix provide clarity over what PHARMAC considers when it makes a funding decision?
4. Should the decision-making matrix be applied to all PHARMAC's decisions including Schedule, Named Patients and implementation decisions? Why or why not?
5. Are there other dimensions that you would include? Are there any dimensions that you would leave out? Why?
6. What alternatives to the proposed decision-making matrix could PHARMAC use for presenting what it takes into consideration?
7. What factors for consideration could be omitted or what further ones could be included to inform PHARMAC's decisions?

2.4 Place the decision-making matrix within the broader framework that PHARMAC operates within

A recommendation made by a large number of submitters in the consultation was to include an overarching set of principles to preface the criteria, in order to establish the limits and boundaries within which PHARMAC operates. We have taken this recommendation on board and propose to set out our broader operating framework, which is shaped by our legal obligations and other guiding documents.

Below is a diagram of the overarching framework. PHARMAC is established under the New Zealand Public Health and Disability Act 2000 and is a Crown entity under the Crown Entities Act 2004, but the framework also includes the wider legal framework and guiding documents.

The framework also explicitly acknowledges the principles that guide the Government's Medicines New Zealand Strategy. The Strategy was developed in 2007 with the purpose of providing an overarching policy direction to align the medicines sector and the systems that govern the regulation, procurement, management and use of medicines. These form part of PHARMAC's broader high-level framework and therefore play a role in shaping our decision-making processes.

This broader operating framework relates to our decision-making in three ways. Firstly, the framework identifies the scope within which all of PHARMAC's operations, including its funding decisions, must operate. Secondly, it helps to inform the development of our factors for consideration and the processes that support our decision-making. Thirdly, it can help us to interpret and clarify the intent and purpose of our factors for consideration in situations of ambiguity.

This framework guides not only our decision-making processes and procedures but all of our OPPs, and therefore we propose to include this at the front of PHARMAC's OPP document (Appendices 3 and 4 show the current and proposed changes to the decision criteria section of the OPP).

PHARMAC's legal framework and guiding documents

<table>
<tr><td>PHARMAC's establishing legislation:</td><td colspan="2">New Zealand Public Health and Disability Act 2000 and Crown Entities Act 2004</td></tr>
<tr><td>Wider legal framework:</td><td colspan="2">This includes: administrative law obligations, Principles of Treaty of Waitangi, New Zealand Bill of Rights Act 1990, Human Rights Act 1993, Official Information Act 1982, Privacy Act 1993</td></tr>
<tr><td>Accountability documents:</td><td colspan="2">This includes: statement of intent, output agreement, letter of expectations</td></tr>
<tr><td>Guiding documents:</td><td colspan="2">This includes: Medicines New Zealand Strategy principles, public sector procurement guidelines</td></tr>
<tr><td colspan="3">Statutory objective: to secure for eligible people in need of pharmaceuticals the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided</td></tr>
<tr><td rowspan="2">Rest of OPPs</td><td>Factors for Consideration</td></tr>
<tr><td>Supporting Information</td></tr>
</table>

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?

2.5 Broaden the scope in relation to populations with health disparity

Health disparity within Māori and Pacific peoples populations is currently taken into account through criterion 2 (the particular health needs of Māori and Pacific peoples). However submitters questioned the purpose and meaning of this criterion.

As it currently stands, criterion 2 serves two purposes; it acknowledges the place of the Treaty of Waitangi in New Zealand, and it also acknowledges the health disparity that currently exists within Māori and Pacific peoples populations.

In relation to the second purpose, we acknowledge that health disparity is not unique to just Māori and Pacific peoples population groups and therefore propose to broaden the scope of this factor for consideration. We propose the following wording for this factor:

> *The impact on the health outcomes of population groups experiencing health disparities including Māori and Pacific peoples*

The intention of this wording is to ensure that, where applicable, PHARMAC considers whether a funding decision may improve the health outcomes of those population groups with a health disparity relative to the rest of the New Zealand population. We propose to retain the specific

reference to Māori and Pacific peoples to acknowledge the significant health disparity that remains evident within these population groups.

'Population groups' in this context refers to specific populations living in New Zealand defined in terms of geographical, demographic, ethnic or socioeconomic characteristics (i.e. not defined by illness or health status characteristics, because health need is considered under a separate factor for consideration).

At the same time, by explicitly including the Treaty of Waitangi as part of the broader operating framework in (as shown in the diagram on page 11), PHARMAC would acknowledge the important place of the Treaty within the wider context in which PHARMAC operates. We have also reflected the Principles of the Treasury within the factors for consideration.

10. What would be achieved by broadening the health disparity factor to include any population groups experiencing health disparities?
11. How is the Treaty of Waitangi best reflected in the proposed framework? Why?

2.6 Remove current criterion 9

We propose to remove current criterion 9 ("Such other criteria as PHARMAC thinks fit) because it is not needed in the proposed factors for consideration decision-making matrix.

In the consultation, some submitters felt that it was critical to keep this criterion so that PHARMAC retains the flexibility to include other relevant factors if required. However, the proposed new decision-making matrix and reframing of most of the existing 'criteria' as 'factors for consideration' confirms for the discretion that PHARMAC has in taking into account all relevant factors in a given decision, even those that have not been anticipated.

It is important to note that if appropriate PHARMAC would still carry out consultation, if PHARMAC intended to consider an additional factor in relation to a specific decision. We propose to include a statement to this effect in the appropriate section of the OPPs, and this may also be considered further when the consultation section of the OPPs is reviewed in due course.

12. What would be the impact of removing the current decision criterion 9 ("such other criteria as PHARMAC thinks fit)?

2.7 Better express the meaning of the proposed factors for consideration and develop supporting information to provide additional clarity and explanation

With PHARMAC's role having significantly expanded since the criteria were initially developed, it is proposed that the wording of the current criteria be revised to reflect that medical devices are now explicitly captured. In addition, consultation feedback clearly demonstrated that the current wording is at times unclear. In developing the factors for consideration, we have attempted to be more clear and explicit about what may be taken into account in a given decision.

To further supplement the factors for consideration, we have also developed a supporting information document, as recommended by a number of submitters. The intention of this document is to provide more detailed explanation of each of the factors (attached as Appendix 5). The document itself will not form part of the OPPs and is for illustrative purposes only.

We propose this would be a living document that would be updated as required. For example as it becomes apparent how specific factors for consideration may be taken into account for medical devices, further explanation and examples where appropriate could be included in the supporting information to reflect this.

13. What is your view on the proposed rewording of the factors for consideration?
14. Which factors, if any, are unclear or confusing?
15. How helpful would the inclusion of a supporting information document be? How would the draft document (appendix 5) provide more clarity and transparency?

3. Recommendations from the consultation not included in this proposal

There were some recommendations made by submitters in the consultation that were considered by PHARMAC but not included in this proposal for change. The key recommendations are discussed in detail below and additional commentary on other key themes that emerged in the consultation has been included in the table attached as Appendix 2.

3.1 Separate criteria for medical devices

In response to the consultation questions we posed regarding medical devices, it was apparent that there are a number of substantive differences that need to be taken into account with respect to medical devices, separate to medicines. This could include, for example, the additional costs for patients associated with some devices as well as wider system costs such as maintenance, training and consumable costs. There were mixed opinions from submitters as to whether these differences required separate criteria, or if they could be captured within the current criteria.

In developing the new proposed framework for decision-making we acknowledge the substantive differences between devices and medicines, but also recognise the importance of maintaining consistency across all our funding decisions. Thus we consider that the proposed framework and rewording of the factors for consideration effectively captures what may need to be taken into account for both medicines and medical devices. Using the example of cost as above, the new decision-making matrix shows how cost may be considered at the patient level, treatment level and the wider health sector level and we note that there are likely to be a much broader range of costs that need to be considered when PHARMAC is considering a medical device rather than a medicine. There are no relevant needs benefits, costs or suitability issues related to medical devices (such as maintenance, consumables and additional training requirements) that we consider cannot be captured within the proposed framework.

It is important to note that as PHARMAC is still in the process of establishing how exactly medical devices will be managed; further detail on what may be taken into account in a given

funding decision cannot yet be provided. However, feedback is specifically invited on whether this new decision making matrix captures the evident differences with medical devices, if there are still other considerations that have been missed.

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?

3.2 Inclusion of community values

We specifically asked in our consultation if our criteria reflect fairness or 'community values,' and many submitters felt that these concepts were not well reflected in the current criteria. Other submitters discussed the challenges of defining community values and the potential disadvantage that could result in PHARMAC making these types of judgements.

The feedback provided has confirmed that 'community values' would be very difficult to include as a specific factor for consideration in and of itself. Reflecting on the discussion during the consultation around community values and ethics, and the moral principles of fairness and equity, we have identified a number of issues that would be encountered if we were to establish some or all of these as specific factors for consideration:

- There are a range of ethical frameworks that PHARMAC could use, and different people subscribe to different frameworks.
- Our statutory objective by referencing "best health outcomes" implicitly reflects a "consequentialist" ethical approach, i.e. one in which actions are judged by their consequences. This means our freedom to choose between competing ethical frameworks is constrained to a degree, if we are to operate lawfully.
- Fairness also means different things to different people. PHARMAC's approach – which seeks "best health outcomes" but within a framework shaped by the decision criteria – reflects one conception of "fairness". Again, our statutory objective is the touchstone for all decisions.
- New Zealand does not consist of a single homogeneous community and therefore does not hold a single set of "community values". There are a range of values, by definition conflicting, held by different individuals and communities.

We consider that the proposed new decision-making matrix better captures the judgement that is required when determining what 'best health outcomes' are, and seek your further feedback on whether it adequately captures what is important to New Zealanders.

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?

3.3 Broader environmental and socioeconomic considerations

Several 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 medical device, and asked that it consider whether there are alternative products or companies that have a lower environmental impact.

Some submitters also thought non-health government priorities should be considered by PHARMAC, because PHARMAC is operating on behalf of the taxpayers of New Zealand and should not be narrowly focussed on the health budget.

PHARMAC is required to operate within the scope of its statutory objective: "to secure for eligible people in need of pharmaceuticals the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided". There is a risk of acting inconsistently with this statutory objective if such broader considerations are taken into account substantially when PHARMAC makes decisions.

Factoring in the impact of PHARMAC's decisions on non-health objectives also raises a number of theoretical and practical issues including:

- ethical and legal considerations – e.g. should the needs of paid workers be valued more highly than children/elderly people? To do so may well be considered discriminatory under the Human Rights Act 1993;
- differences in assumptions used by other government agencies resulting in inconsistent analyses; and
- the difficulty and cost of PHARMAC accurately estimating the impacts of potential decisions to areas outside the health sector;

Environmental impacts can result in health benefits, costs and / or risks. In instances where this is the case, the health outcomes can and should be considered as part of PHARMAC's decision making framework, to the extent that this is possible.

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?

3.4 The consideration of treatments for rare diseases

Many submitters considered that current criteria adversely affect the chances of high cost and orphan drugs being funded. These submitters believed more weighting should be given to patients with rare diseases through additional criteria or differentiated criteria under the Named Patient Pharmaceutical Assessment (NPPA) policy. Many submitters also emphasised the need for flexibility in cases of rare and complex conditions, particularly in situations where patients face a poor prognosis for survival or prolonged pain and suffering without access to a particular treatment or device.

We note and acknowledge that rare conditions are often very serious and debilitating conditions. The severity of the illness, and consequently the health need of the patient population, is a very

important consideration, and this is reflected through the proposed 'need' dimension in the factors for consideration matrix, and in particular, the 'health need of the patient population' factor for consideration.

The fact that a pharmaceutical treats a rare condition does not, in itself, disadvantage it in the current assessment process, nor in the proposed decision-making matrix. PHARMAC's assessment currently determines the value offered by a medicine relative to its cost, regardless of the size of the population group that stands to benefit. If the population in which the medicine is effective is small (either because the medicine is targeted to a subset of a disease group or because the disease itself is rare) the value and cost is assessed for this patient group (not the broader population).

We consider that the use of the same decision-making matrix for all applications for treatments for named patients and for population groups helps to ensure the fair and consistent consideration of these applications. We consider there is nothing about the situations described by submitters that warrant the inclusion of a new factor for consideration related to rarity.

We recognise that it can sometimes be difficult for suppliers to obtain clinical trial data when the target population group is small, and that as a consequence the quality of the clinical evidence may be poorer when the condition is particularly rare. This, however, relates to 'how' PHARMAC considers each of the relevant factors relative to each other, and how we make decisions with incomplete or uncertain information. This point highlights the need for flexibility in decision making, and the ability for PHARMAC to use judgement to consider complex or more unusual situations. It is partly for these reasons that we have proposed re-framing the current decision criteria as factors for consideration.

21. How well does the proposed approach adequately address the considerations that are relevant to funding proposals for treatments for rare diseases? Why?

Appendix 1: Consultation questions

1. How helpful is a high-level summary in better explaining what PHARMAC takes into account?
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?
3. How would the presentation of a decision-making matrix provide clarity over what PHARMAC considers when it makes a funding decision?
4. Should the decision-making matrix be applied to all PHARMAC’s decisions including Schedule, Named Patients and implementation decisions? Why or why not?
5. Are there other dimensions that you would include? Are there any dimensions that you would leave out? Why?
6. What alternatives to the proposed decision-making matrix could PHARMAC use for presenting what it takes into consideration?
7. What factors for consideration could be omitted or what further ones could be included to inform PHARMAC’s decisions?
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?
10. What would be achieved by broadening the health disparity factor to include any population groups experiencing health disparities?
11. How is the Treaty of Waitangi best reflected in the proposed framework? Why?
12. What would be the impact of removing the current decision criterion 9 (“such other criteria as PHARMAC thinks fit)?
13. What is your view on the proposed rewording of the factors for consideration?
14. Which factors, if any, are unclear or confusing?
15. How helpful would the inclusion of a supporting information document be? How would the draft document (appendix 5) provide more clarity and transparency?
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?
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?
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?
21. How well does the proposed approach adequately address the considerations that are relevant to funding proposals for treatments for rare diseases? Why?

Appendix 2: Feedback table

SUBMISSION COMMENT	PHARMAC RESPONSE AND PROPOSED POLICY ACTION
1 What are your views on the value of the current nine decision criteria?	
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 suggested that if any change is needed, it may lie in continuing to increase public understanding of the process and how it works.	Noted. We recognise the importance of consistency in decision-making, and our proposal endeavours to improve the public's understanding of what we take into account when we make a funding decision, and also the broader operating environment that establishes the limits and boundaries we operate within.
A number of submissions 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 (OPPs).
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.	Our proposal attempts to frame our decision making within PHARMAC's broader operating framework, which includes legislation such as the Human Rights Act. Refer to section 3.4 in the body of the consultation document for a more detailed discussion about rare disorders.
2 What have been your experiences with our current decision criteria?	
A small number of submissions reported on positive outcomes; however a large number of submitters reported negative experiences, namely in relation to PHARMAC not funding specific pharmaceuticals. 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.	It is useful to receive feedback in relation to individual funding decisions and we have taken this into consideration, although it is not possible to respond to each one in this consultation response. As noted above, feedback about the process through which PHARMAC applies the decision criteria is outside the scope of this review, but will be taken into consideration and fed into upcoming reviews of other aspects of the OPPs.

SUBMISSION COMMENT	PHARMAC RESPONSE AND PROPOSED POLICY ACTION
Many of the submitters who reported negative experiences commented that they felt the fiscal impact of the funding decision outweighed the consideration of other criteria.	As PHARMAC is bound by legislation not to exceed the funding available, the cost of a treatment will always be an important consideration. However, it is only one of a number of considerations. This is hopefully made clearer by the proposed decision-making matrix.
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.	How the criteria are used and applied is beyond the scope of this review, however we hope that the proposed decision-making matrix makes what we take into account more transparent.
3 To what extent should the criteria give PHARMAC the flexibility to make decisions on a case-by-case basis, and to exercise judgement?	
Many submitters acknowledged the need for criteria to be flexible; this was noted specifically in consideration of rare or complex conditions.	Noted. This feedback has informed our proposal to reframe the criteria as factors for consideration.
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 to reduce the risk of political pressure interfering with decision-making.	Noted. We have attempted to achieve a balance between flexibility and specificity.
A number of people recommended some approaches to exercising flexibility, for example, having a broader set of general criteria with layers of specificity below this.	This feedback has informed our decision to frame the factors for consideration in terms of the broader strategic considerations, and to provide a greater degree of specificity in the 'supporting information' document.
4 Is there anything about the nine existing criteria that make them inappropriate to be applied to medical devices? Why?	
Most submitters felt amendment to the criteria would be required to encompass devices. Various submitters across different submitter-types considered the current criteria inappropriate, given devices are fundamentally different from pharmaceuticals; for example, medical devices include capital expenditure and maintenance.	We have noted and acknowledge the ways in which devices are fundamentally different from pharmaceuticals, and this is partly why we have included a 'suitability' dimension within the proposed decision-making matrix. We consider that all of the considerations relevant to medical devices (for example, maintenance costs) are able to be captured (refer section 3.1 for further discussion)

SUBMISSION COMMENT	PHARMAC RESPONSE AND PROPOSED POLICY ACTION
Several submitters specifically noted the inappropriateness of the current criteria 5 (cost-effectiveness) for medical devices. This was particularly in relation to the additional costs that need to be taken into consideration for medical devices. 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.	We note the importance of a range of different considerations with regard to medical devices, such as product life cycle, surgical skill etc, and have attempted to create a decision-making matrix that is able to capture these types of considerations. Cost-effectiveness, however, remains relevant to our decisions, because PHARMAC's statutory objective is to secure for eligible people in need of pharmaceuticals the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided. More detail about what specific issues PHARMAC will think about when applying the factors for consideration to Medical Devices will be provided in due course.
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).	We note that PHARMAC may often need to make funding decisions in the absence of full or perfect information, but believe that the proposed framework is flexible enough to accommodate such situations.
Other risks identified were that implementation of the current decision criteria would not acknowledge the importance of change management in relation to medical devices. One 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 medicine.	The proposed decision-making matrix makes it clear that PHARMAC considers impacts to the patient and health sector, and considers suitability considerations (which could include, for example, the cost and implications of change. We therefore consider the proposed decision-making matrix would enable us to consider these device related considerations.
5 What other criteria might be needed when considering the priority of a medical device?	
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. 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	We agree that the initial purchase cost of a device is unlikely to be the only relevant consideration, and have attempted to capture that in our proposed approach.

SUBMISSION COMMENT	PHARMAC RESPONSE AND PROPOSED POLICY ACTION
consideration, as well as direct and indirect patient and other stakeholder benefits.	
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.	Noted, we have attempted to capture these considerations in the 'clinical benefits and risks of the medicine or medical device' factor in the decision-making matrix.
Usability: Some submitters and a number of attendees at community forums considered usability of the device needed to be a criterion.	We have added a 'suitability' dimension to the decision-making matrix.
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.	We have included a 'need' dimension in the decision-making matrix.
Assessment in relation to supplier: a number of submitters commented on the relevance of support and services offered by device suppliers	We agree that the support and services offered by device suppliers are likely to be relevant considerations, and we anticipate that under the proposal, these aspects would be considered as part of mitigating the potential 'risks' of the medical device. We note that supplier reputation is already currently a 'matter for evaluation' in our annual Tender.
Disinvestment: Several submitters referred to criteria for making disinvestment decisions relating to medical devices.	While most of our decisions are to improve access or make new investments, rather than to disinvest, we note that the considerations should be the same i.e. decisions to disinvest need to be made for the purposes of achieving the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided. For this reason, we consider that the decision making framework proposed should apply to all funding related decisions, including decisions to disinvest. The issue of disinvestment decisions with respect to medical devices in particular will be considered in more detail during the medical devices establishment consultation process.

SUBMISSION COMMENT	PHARMAC RESPONSE AND PROPOSED POLICY ACTION
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.
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?	
Submitters identified advantages as being comparative allocation of limited resources, comparison of cost/benefit data for pharmaceuticals and devices, and accommodating difference within common criteria.	Noted, this feedback has informed our proposal to use the same factors for consideration for both medicines and medical devices.
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.	We have tried to capture the various stakeholder dimensions in the proposed decision-making matrix. We consider we have been able to capture all of the considerations relevant to medical devices in the decision-making matrix, but would appreciate further feedback on this.
Submitters identified comparative complexity of devices as a disadvantage.	Although we acknowledge that devices are often more complex than medicines, we have attempted to capture this complexity in the proposed factors for consideration. The 'suitability' dimension, for example, is likely to be less relevant for medicines than for medical devices, but we consider the proposed approach provides enough flexibility for PHARMAC to consider the aspects that are most relevant for the particular decision under consideration.
7 How specific should the criteria be? How general should they be?	
Respondents who considered criteria should be as general as possible felt the criteria should offer guidance not prescription, and that they should be flexible with specificity occurring at the next level down.	We have attempted to make it clear that PHARMAC retains flexibility by proposing to reframe the criteria as factors for consideration. The proposed supporting information document is intended to provide more detailed guidance.
Those respondents who considered that criteria should be as specific as possible felt this would provide greater clarity and transparency.	We have tried to make the proposed factors for consideration in plain language and as clear and specific as possible; however, there is a need to strike a balance between flexibility

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	and specificity.
8 What other criteria should/could PHARMAC consider?	
Submitters across different submitter-types suggested a number of general considerations that should apply to whatever set of criteria is used. These include: consistency in decision-making, reflecting community values, incorporating consumer input, ensuring legal obligations are met, and the principles of fairness and equity.	'Consistency in decision making' and 'incorporating consumer input' refers to process rather than the criteria themselves, and these are therefore outside of the scope of this review, but will be taken into consideration during future reviews of the rest of the OPPs. Refer to section 3 of this consultation document for a more detailed discussion of some of the other proposals.
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 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.	Refer to section 3.4 for a detailed response on these issues.
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.	Noted. We have attempted to capture all of the types of considerations that may be relevant to achieving our statutory objective. We welcome further feedback as to whether there is anything that the proposed approach has not adequately captured.
9 Of the current criteria, which remain appropriate to retain? Why? Which ones are no longer appropriate? Why?	
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. 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	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 (OPPs).

SUBMISSION COMMENT	PHARMAC RESPONSE AND PROPOSED POLICY ACTION
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.	We have attempted to make the proposed factors for consideration more transparent through wording changes and the proposed supporting information document.
10 If you were to have a clean slate, around what criteria would you base decisions for funding pharmaceuticals within a fixed budget?	
Many submitters made recommendations about having an overarching set of principles to preface the criteria.	This feedback has informed our proposal set out PHARMAC's broader operating framework as part of the document.
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.	The proposed amendments to the current criteria are intended to address this issue.
11 How do the criteria currently reflect fairness or community values?	
The principles of fairness and equity were discussed by a number of submitters, who felt the criteria needed to take into account the health needs of the population as a whole, as well as sub-groups and individuals.	Refer to section 3.2 for a detailed response on these issues.
It was also stated that criteria should reflect patients' rights, including under New Zealand law and international obligations.	
Several submitters, including a professional association and a clinician, stated that the current criteria broadly reflect fairness.	
Submitters also expressed general concerns related to consultation processes, the lack of commonly accepted conceptions of fairness or community values, equity rather than fairness is important, and that the process rather than the criteria require amendment.	
12 What additional criteria would you suggest to reflect fairness or community values and how could these be measured?	
Most—but not all—submitters who answered this question 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.	Refer to section 3.2 for a detailed response on theses issues.

SUBMISSION COMMENT	PHARMAC RESPONSE AND PROPOSED POLICY ACTION
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.	
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.	
13 What additional information or detail do you think should be included in the decision criteria section of the OPPs?	
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.	We have attempted to do this through the proposed supporting information document and high level operating framework and principles.
Other suggestions were clarification of the review process, non-Schedule applications, specification of the criteria that apply to pharmaceuticals and/or devices, establishing decision criteria in relation to new areas of business.	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 (OPPs).

Appendix 3: Current Decision Criteria Chapter of PHARMAC's OPPs

2.2 Decision Criteria

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:

j) the health needs of all eligible[2] people within New Zealand;

k) the particular health needs of Maori and Pacific peoples;

l) the availability and suitability of existing medicines, therapeutic medical devices and related products and related things;

m) the clinical benefits and risks of pharmaceuticals;

n) the cost-effectiveness of meeting health needs by funding pharmaceuticals rather than using other publicly funded health and disability support services;

o) the budgetary impact (in terms of the pharmaceutical budget and the Government's overall health budget) of any changes to the Schedule;

p) the direct cost to health service users;

q) 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

r) such other criteria as PHARMAC thinks fit. PHARMAC will carry out appropriate consultation when it intends to take any such "other criteria" into account.

[2] As defined by the Government's then current rules of eligibility.

Appendix 4: Proposed 'Factors for Consideration' Chapter in PHARMAC's Operating Policies and Procedures

2.2 Factors for Consideration

PHARMAC uses the factors for consideration set out in this clause to inform decisions about proposed amendments to the Schedule and decisions outside the Schedule relating to treatments for named patients. The purpose of the factors is to assist PHARMAC to determine whether any decision or proposal helps PHARMAC to achieve its statutory objective. The extent to which any one particular factor is relevant, if at all, and the weight to be given to each factor is for PHARMAC to determine on each occasion. Where PHARMAC makes decisions that do not involve amendments to the Schedule (for example, decisions relating to the promotion of the responsible use of medicines), it endeavours to use these factors, to the extent that they are relevant to those decisions. If PHARMAC takes into account a factor that is additional to the "factors for consideration" listed below in making a decision, it will consult on the use of that additional factor where it is appropriate to do so. The factors for consideration should be considered within the context of the broader legal framework within which PHARMAC operates, as set out below:

PHARMAC's legal framework and guiding documents

PHARMAC's establishing legislation:	**New Zealand Public Health and Disability Act 2000 and Crown Entities Act 2004**
Wider legal framework:	**This includes: administrative law obligations, Principles of Treaty of Waitangi, New Zealand Bill of Rights Act 1990, Human Rights Act 1993, Official Information Act 1982, Privacy Act 1993**
Accountability documents:	**This includes: statement of intent, output agreement, letter of expectations**
Guiding documents:	**This includes: Medicines New Zealand Strategy principles, public sector procurement guidelines**
Statutory objective: to secure for eligible people in need of pharmaceuticals the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided	
Rest of OPPs	Factors for Consideration
	Supporting Information

Factors for consideration

	Need	Benefits	Costs	Suitability
Patient	The health need of the patient population under consideration relative to all eligible people within New Zealand	Clinical benefits and risks of the medicine or medical device to the patient and health outcomes	Out of pocket costs to the patient using the medicine or medical device	Suitability of the medicine or medical device to the patient
	The Principles of the Treaty of Waitangi			
	The impact on the health outcomes of population groups experiencing health disparities including Māori and Pacific peoples			
Treatment	The availability and suitability of existing medicines, medical devices and treatments for the clinical use under consideration		Cost of the medicine or medical device	
Health Sector	Supporting Government health priorities	Benefits and risks to the health sector of the medicine or medical device	Flow-on costs of the medicine or medical device to the rest of the health sector	Suitability of the medicine or medical device to the health sector

Statutory objective:

Does the proposal or decision help PHARMAC to secure for eligible people in need of pharmaceuticals the best health outcomes that are reasonable achievable from pharmaceutical treatment and from within the amount of funding provided?

The factors for consideration are supplemented by a 'supporting information' document that provides more information about what the factor dimensions mean and what they include. This supporting document does not form part of the OPPs and is provided for illustrative purposes only.

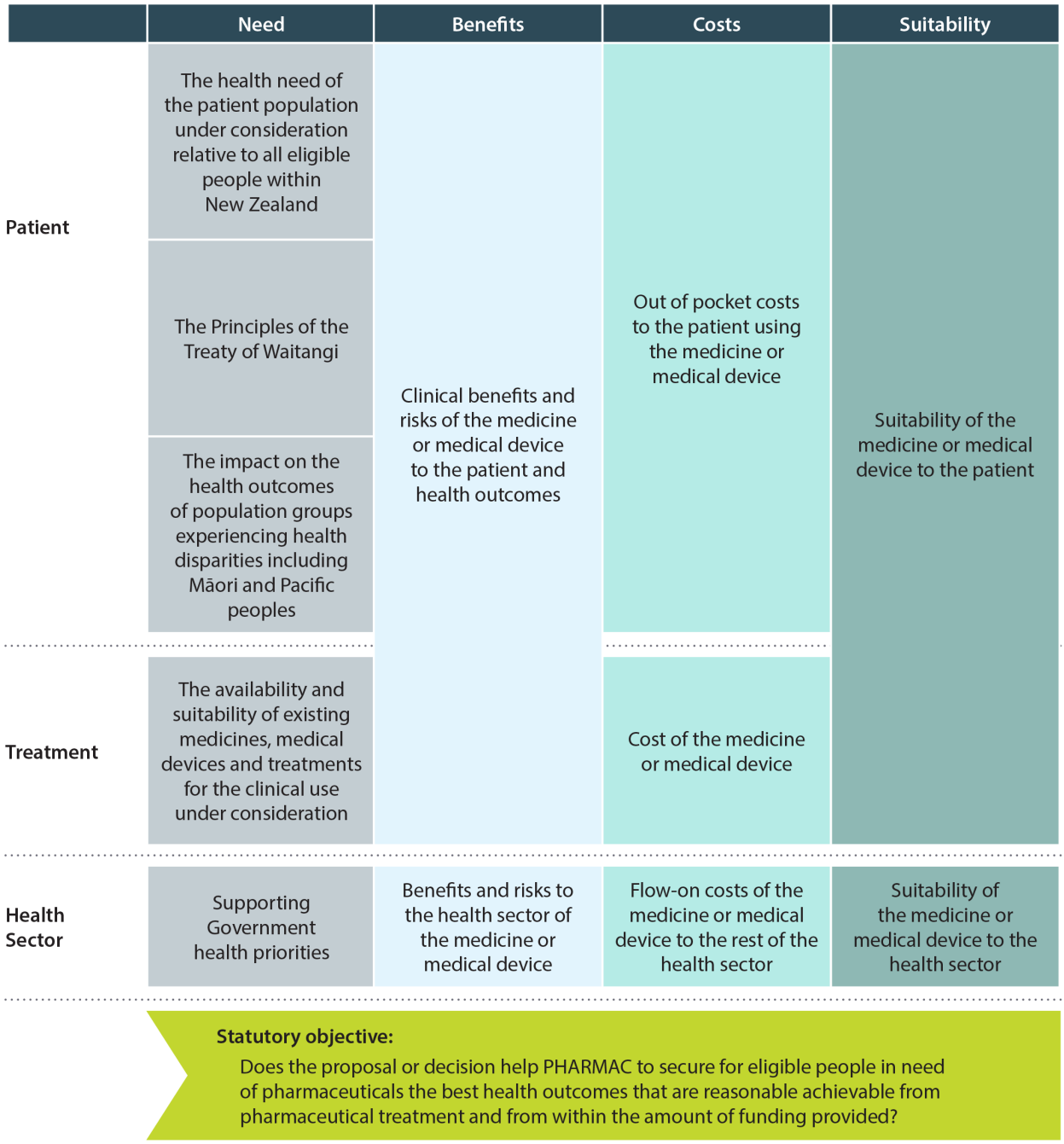
Named Patient Pharmaceutical Assessment (NPPA) decisions for individual named patients and Pharmaceutical Tender decisions also have supplementary considerations that provide an additional level of specificity:

- NPPA applications: Prior to the above factors being taken into consideration, NPPA funding applications must meet specified prerequisites as outlined in the NPPA policy.
- Tender decisions: Additional factors are annually consulted on and published in the ‘Matters for evaluation’ section of the annual Invitation to Tender document.

Appendix 5: Supporting Information

This document provides an additional explanation of the factors for consideration that PHARMAC uses to decide whether any decision or proposal helps PHARMAC to achieve its statutory objective. The factors for consideration are used to inform decisions about proposed amendments to the Pharmaceutical Schedule and decisions outside the Schedule relating to treatments for Named Patients. Where appropriate PHARMAC also endeavours to use these factors when making decisions that do not involve amendment to the Schedule (for example decisions relating to the promotion of the responsible use of medicines), to the extent they are relevant to these decisions. This document does not form part of the OPPs and is provided for illustrative purposes only.

The diagram below shows how the factors for consideration fit together within a decision-making matrix.



Each of the factors for consideration is set out below and, where possible, examples have been given to provide further clarification. We have also noted, in relation to some of the factors, what PHARMAC does not intend to take into account.

Factors for consideration help inform our consideration of whether a decision will achieve our statutory objective of securing for eligible people in need of pharmaceuticals the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided.

PHARMAC uses the factors for consideration where applicable and giving such consideration to each factor as PHARMAC considers appropriate. It is important to note that some factors may be more or less relevant depending on the type and nature of the decision being made and, therefore, judgement is always required. The ability to exercise this judgement is critical to PHARMAC's role as it enables us to respond appropriately to a broad range of situations.

In addition, PHARMAC works within a fixed budget (Community Pharmaceutical Budget – CPB), and this budget changes from year to year. We also have an indicative future 3 year funding path to allow us to make investment decisions on pharmaceuticals and be sure we can still afford those pharmaceuticals in the future. Therefore the factors for consideration described below may have more or less relevance depending on the circumstances that are relevant at the time. Therefore there is no simple or specified calculation used to apply the factors as this is simply not possible.

However, all decisions being made by PHARMAC within our OPPs are made within PHARMAC's broader operating framework as set out below.

PHARMAC's legal framework and guiding documents

PHARMAC's establishing legislation:	**New Zealand Public Health and Disability Act 2000 and Crown Entities Act 2004**
Wider legal framework:	**This includes: administrative law obligations, Principles of Treaty of Waitangi, New Zealand Bill of Rights Act 1990, Human Rights Act 1993, Official Information Act 1982, Privacy Act 1993**
Accountability documents:	**This includes: statement of intent, output agreement, letter of expectations**
Guiding documents:	**This includes: Medicines New Zealand Strategy principles, public sector procurement guidelines**
Statutory objective: to secure for eligible people in need of pharmaceuticals the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided	
Rest of OPPs	Factors for Consideration
	Supporting Information

This document is a dynamic document that will be updated as required to reflect changes or to link to new information that may provide additional clarification. Given PHARMAC is only in the preliminary stages of determining how medical devices will be managed, the document may need to be updated to reflect decisions in relation to medical devices. It has been noted below where additional considerations specific to medical devices may be taken into account in due course. At this stage this supporting information only provides an indication of what may be included. You can find more information about PHARMAC's medical devices establishment work here.

The health need of the patient population under consideration relative to all eligible people within New Zealand

Health need

'Health need' refers to the overall health status of the patient group that would be treated with the medicine or medical device we are considering. We consider the patient group's health status as a result of the illness, without considering what impact the treatment might have on that health status (which is considered under other factors).

In order to compare relative health needs, PHARMAC currently uses a metric where possible to describe the gap in average health status between those in the patient group and those at full health. The metrics firstly define a given disease or condition and the prevalence (numbers) of patients with that disease according to age and sex (if this data is available). The metrics then present health status information for individuals with that disease, including both life expectancy and quality of life. Gaps (absolute and relative) between the level of health status of those with the disease/condition under standard care and those in full health are then used as measures of the level of health need.

The health status metrics describe and compare differences in life expectancy and quality of life, which PHARMAC can take into account when making funding and implementation decisions. The metrics do not show the benefits of a particular treatment under consideration (or the patient's capacity to benefit), nor indicate how 'best' to distribute health gains. These considerations are taken into account in other factors.

Eligibility

The Health and Disability Services Eligibility Direction 2011 sets out the eligibility criteria for publicly funded health and disability services in New Zealand. The direction is made by the Minister of Health under section 32 of the New Zealand Public Health and Disability Services Act 2000. The direction became effective on 16 April 2011, and applies from that date forward

For more information about the groups of people who meet the criteria defined in the Direction and can receive some or all publicly funded health and disability services, see: http://www.health.govt.nz/new-zealand-health-system/eligibility-publicly-funded-health-services

This factor for consideration is not intended to include:

- The patient group's capacity to benefit from the proposed treatment (considered under clinical benefits and risks of the medicine/medical device to the patient and suitability of the medicine/medical device);
- The efficacy of the proposed treatment (considered under other factors for consideration)

The Principles of the Treaty of Waitangi

PHARMAC acknowledges the special relationship that exists between the Crown and Māori and recognises the articles of the Treaty of Waitangi expressed through the principles of Partnership, Protection and Participation.
Partnership – forging and maintaining enduring relationships with whānau, hapū and iwi.
Protection – ensuring Māori have the same access to medicines as non-Māori and receive at least the same level of health outcomes through advancing tino rangatiratanga.
Participation – respecting and trusting each other's ability and knowledge about how best to do the work to achieve shared outcomes.

The impact on the health outcomes of population groups experiencing health disparities including Maori and Pacific peoples

This factor enables PHARMAC to consider specific populations in New Zealand where there is a substantive discrepancy between the health of the specified population group relative to the rest of the New Zealand population. This relative health disparity may be the result of an underlying disadvantage (rather than an illness), which is a separate consideration to the absolute 'health need' of a given patient group as discussed in the above factor (health needs of the patient population).

PHARMAC has acknowledged the health disparity that continues to be prevalent amongst Māori and Pacific peoples. PHARMAC's Te Whaioranga Strategy and the Pacific Responsiveness Strategy have been developed and implemented to help specifically address the health issues faced by these population groups. However, PHARMAC acknowledges that health disparity is not limited to just Māori and Pacific peoples, and, where appropriate, health disparities faced by other specific populations will be taken into account when funding and implementation decisions are made.

Population groups
'Population groups' refers to specific groups living in New Zealand, such as Māori and Pacific peoples populations, defined in terms of geographical, ethnic, demographic or socioeconomic characteristics i.e. not defined by illness or health status characteristics, because health need is considered under the factor above (health needs of the patient population.) This may also include, but not be limited to, populations groups outlined on the Ministry of Health website: http://www.health.govt.nz/our-work/populations.

This factor for consideration is not intended to include:

- Patient groups defined by a specific illness

The availability and suitability of existing medicines, medical devices and treatments for the clinical use under consideration

This factor considers the other medicines, medical devices and treatments that are currently available in New Zealand for the relevant population group, and assesses their effectiveness in relation to the proposed treatment.

Availability

Existing medicines: generally refers to medicines that are currently listed on the Pharmaceutical Schedule or otherwise publicly funded.

Medical devices: until the time when PHARMAC has full management of devices, *existing medical devices* is unlikely to be limited to only what is currently funded/ managed by PHARMAC. PHARMAC is still considering what would be defined as an available existing medical device for the interim period. It is likely that a medical device would be considered 'available' if it is used as part of standard practice within DHB hospitals[3] or the community.

Treatments: may refer to any other relevant publicly funded treatment or service such as physiotherapy or surgery. A treatment will generally be considered 'available' if it is used as part of standard practice within DHB hospitals (that provide the relevant treatment or service) or the community.

Suitability

Suitability refers to the appropriateness and proven effectiveness of the available medicines, medical devices or other treatment options, relative to the proposed treatment.

PHARMAC is continuing to consider how it will assess the 'suitability' of medical devices. However, relevant factors that could determine an alternative's suitability may include, for example ease of use, the required clinician skill and training, patient acceptability and the overall impact of change on clinicians and patients, insofar as these considerations may impact on health outcomes.

This factor for consideration is not intended to include:

- Preference for a given medicine or medical device where this preference does not impact on ease of use or compliance and consequently effectiveness (i.e. where there is no tangible impact on health outcomes). PHARMAC acknowledges that with medical devices, clinical preference can impact on uptake and effectiveness and therefore ultimately health outcomes. In these instances where a tangible health outcome is evident, 'preference' may be considered relevant.

Government health priorities

As a Crown entity, PHARMAC is directly accountable to the Minister of Health who is ultimately accountable for PHARMAC's performance. PHARMAC must give effect to a direction set by the responsible Minister or a whole of Government direction, when such directions are given in accordance with the Crown Entities Act (s103-115). However, no direction made by the Minister that would require PHARMAC to purchase a pharmaceutical from a particular source or at a particular price or provide any pharmaceutical or pharmaceutical subsidy or other benefit to a named individual (s65, NZPHD Act).

More information on current Government's priorities for health funding can be found in the following documentation:

- Ministry of Health SOI
- PHARMAC SOI
- Output Agreement
- Letter of Expectations

[3] "DHB Hospital" means a hospital (including community trust hospitals) and/or an associated health service that is funded by a DHB including (but not limited to) district nursing services and child dental services.

This factor for consideration does not take into account:

- Non-health related Government priorities not specifically identified by the Minister of Health as priorities for PHARMAC

Clinical benefits and risks of the medicine or medical device to the patient and health outcomes

Clinical benefits and risks are assessed in relation to how the proposed decision would be expected to affect the health outcome of the individual patient, or in some cases the rest of New Zealand society (for example, in relation to herd immunity benefits to society as a result of vaccination).

Assessing clinical evidence

In determining the clinical benefits and risks of a proposed funding decision, PHARMAC assesses the available clinical evidence, which may include, for example, clinical trials and evidence obtained from use in other jurisdictions. This evidence is assessed relative to currently available alternatives (as described in the factor 'availability and suitability of existing medicines, medical devices and treatments for the clinical use under consideration').

When assessing clinical evidence, PHARMAC considers the strength and quality of the evidence available. This factor is also considered in detail, by PHARMAC's expert advisory committee, the Pharmacology and Therapeutics Advisory Committee (PTAC), and the various specialist sub-committees that sit below PTAC.

Clinical evidence can in some cases be difficult to obtain; for example if there is only a small population size or, if technology is new and therefore clinical trials have not yet been carried out to show long-term effects. In such situations, PHARMAC will consider the lack of evidence, and the risks associated with this, balanced against other relevant factors for consideration such as health need, when making a decision.

Assessing clinical benefits

To determine the cost-effectiveness of a proposed funding/implementation decision, PHARMAC undertakes a cost-utility analysis (CUA), which is the assessment of the benefits and costs associated with a given treatment.

The benefits in a CUA are estimated using 'quality-adjusted life years' (QALYs). QALYs are an internationally used measurement that can be used to compare – in a consistent and standardised way – the benefits of different treatments. Measuring QALYs involves the combination of two major things: a treatment's effects on how much longer a patient may live, and on their quality of life. Examples of quality of life factors that may be taken into account when considering how much better a patient may live include; mobility, ability to self-care, ability to undertake usual activities (e.g. work, leisure), level of pain and discomfort, and anxiety and depression.

Access and Optimal Use

As well as evidence of therapeutic benefits (or risks), PHARMAC also considers if a given decision may have implications for whether patients will gain access to medicines/medical devices, or if there may be any other benefits or risks associated with implementation of a given decision. PHARMAC considers, if relevant, the overall 'cost of change' in relation to the overall disruption to the patient group affected by the potential funding decision. PHARMAC may also take into account clinical benefits and risks that are partially dependent on patient use. For example the vulnerability of the patient group to change may impact on

adherence. Likewise the size, shape and taste of a medicine may impact on patients' adherence to their treatment.

For medical devices, clinical benefits and risks can also be significantly influenced by clinical use, for example if training is required to use a new device effectively. Exactly how and what considerations in relation to clinical use may be taken into account for medical device funding decisions will be determined in due course.

Supplier characteristics

PHARMAC may consider supplier history insofar as this may impact health outcomes for the patient (benefits or risks). This could include, for example, previous experience that PHARMAC may have had with a given supplier in relation to dealing with stock issues. Supplier characteristics are often more relevant for Tender decisions where PHARMAC is considering multiple suppliers for the same product. The Annual Tender document will often include more specific 'matters for evaluation' that PHARMAC considers in relation to the supplier.

Wider population clinical benefits and risks

PHARMAC also takes into account how clinical benefits and risks of a given treatment may have an impact on the wider New Zealand population. This becomes particularly relevant when considering, for example, vaccines where effective vaccination has clinical benefits for the wider population in terms of lowering the risk of the spread of contagious disease.

This factor for consideration is not intended to include:

- Individual or clinical preference for a given medicine or medical device, unless this preference is in some way linked to a clinical benefit or risk or health outcomes.

Benefits and risks to the health sector of the medicine or medical device

PHARMAC considers the benefits and risks to the wider health sector of a given funding decision. It is possible for medicines (and medical devices) to create benefits or save costs elsewhere and therefore have positive impacts for the wider health sector. PHARMAC calls these 'cost offsets', and this is included in the analysis of the relative cost-effectiveness of a proposed medicine or medical device.

For example, freeing up a hospital bed for a treatment that previously required an overnight stay enables the bed to be used by someone else, and therefore has a positive impact in relation to available resources and health outcomes. Or if a treatment previously requiring administration by a health professional is replaced by a self-administered drug; this has benefits for the wider health sector by freeing up the time of the health professional, potentially reducing waiting times.

This factor for consideration is not intended to include:

- Benefits or risks beyond the health sector such as non-health benefits to the justice system or the environment.

Out of pocket costs to the patient using the medicine or medical device

PHARMAC considers the out of pocket expenses that the proposed treatment may have for the patient. These could include both quantitative and qualitative costs. For example PHARMAC may consider the monetary cost to patients of GP visits, pharmaceutical co-payments, home-based care, or continuing care in a rest home or private hospital level geriatric/ psychogeriatric care. PHARMAC may also take into account qualitative costs such as if the patient is required to travel to access a given medicine or medical device.

This factor for consideration is not intended to include:

- The price of the treatment if the patient was to purchase it privately (i.e. if it was not subsidised)
- The cost of patients' time off work (i.e. lost wages) and reduced productivity costs (as this would bias against those not in paid employment such as the elderly and children);
- Costs to non-healthcare government sectors
- Other indirect costs as discussed in the below factor (cost of the medicine or medical device)

Further information on why these factors are not considered are discussed in the Prescription for Pharmacoeconomic analysis (page 46).

Cost of the medicine or medical device

PHARMAC considers the cost of the medicine or medical device relative to the benefits as discussed in the 'clinical benefits and risks' factors above.

Cost analysis

To determine the cost-effectiveness of a proposed funding/implementation decision, PHARMAC undertakes a cost-utility analysis (CUA), which is the assessment of both the benefits and costs associated with a given treatment (benefits are discussed under the 'clinical benefits of the medicine or medical device' factor for consideration').

Costs are carefully considered in CUA. This includes the cost of the treatment itself and any other costs to the health sector that may occur as a result of funding the new treatment. This is discussed further below in the next factor for consideration (flow on costs to the rest of the health sector).

The result of a CUA shows how many QALY's are gained for every dollar spent. This enables PHARMAC to compare treatments for the same conditions, but also to compare the cost-effectiveness of funding treatments for different conditions.

Given the complexity around this particular factor for consideration, PHARMAC has produced a basic explanatory guide, Cost-utility Analysis Explained and also a more detailed and technical document, Prescription for Pharmacoeconomic Analysis (PFPA). The PFPA describes in detail the approach PHARMAC takes when doing cost-utility analysis.

Medical Devices

PHARMAC acknowledges that there are often costs specifically associated with medical devices that differ from those associated with medicines such as the cost of maintenance, possible outsourcing requirements, consumables, transition and training costs and the

compatibility of the device with clinical and IT environments. As we are still in the process of determining exactly what and how these factors should be taken into account, further supporting information in relation to this will be included at a later date.

This factor is not intended to include:

- The cost of patients' time off work (i.e. lost wages) and reduced productivity costs (as this would bias against those not in paid employment such as the elderly and children);
- Cost of premature mortality (as above, this would disadvantage those not working)

Further explanation for these exclusions is included in the PFPA (page 47.)

Flow-on costs of the medicine or medical device to the rest of the health sector

PHARMAC may consider any flow-on costs to the rest of the health sector as a result of the funding decision. Just as funding decisions may have cost-savings to the rest of the health sector (as discussed in relation to the factor, 'clinical benefits and risks of the medicine or medical device to the patient, and health outcomes'); the decision may also result in flow-on costs. For example, a new treatment may be replacing an older treatment that was less time intensive for the clinician. Also, as discussed above, a medical device may involve on-going operational servicing costs.

Suitability of the medicine or medical device to the patient

PHARMAC considers the suitability of a medicine or medical device to the individual patient relative to the currently available treatments when considering a given funding decision. This consideration could include, for example, how easy the product is to use or swallow or the 'cost of change' in relation to the overall disruption to the patient group affected by the potential funding decision.

Suitability of the medicine or medical device to the health sector

PHARMAC considers the compatibility of the medicine or medical device with the rest of the health sector. For example, there may be additional equipment that may be required to administer a treatment, or a proposal may not be compatible with existing best clinical practice.

Statutory objective

The purpose of using the factors for consideration is to assist PHARMAC to determine whether a decision or proposal will help PHARMAC to achieve its statutory objective, which is to secure for eligible people in need of pharmaceuticals the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided. This must be considered by PHARMAC in relation to every funding and implementation decision.

In particular, the factors for consideration help us to determine what 'best' means in the context of best health outcomes. This allows us to consider all of the factors relevant to a

particular decision and then to compare this decision or funding option against all of the possible alternative decisions and funding options, to make a judgement about what set of funding options will secure the best health outcomes that are reasonably achievable.

In addition, PHARMAC works within a fixed budget. Every funding and implementation decision we consider must be considered in relation to how funding the given treatment may impact on the Combined Pharmaceutical Budget (CPB), DHB hospital budgets and the overall Vote Health budget, for both the current financial year and over future years. PHARMAC also considers the likely effects over the coming years by taking into account factors such as market dynamics, for example the impact of a funding decision on future competition in that market.

Although PHARMAC manages the CPB (on behalf of DHBs), we also take into account budgetary impacts on the wider health budget (as discussed in the factors for consideration that explicitly consider health sector impacts).

Budgetary impact is also important for PHARMAC's current role with the Hospital Medicines List (HML) and for Medical Devices, even though there is currently no fixed budget for either area of business. It is still important to consider the financial implications of PHARMAC's investment decisions on DHBs. For HML funding decisions in the interim PHARMAC has agreed with DHBs that PHARMAC's investments in the HML will not exceed savings that PHARMAC has achieved in the hospital medicines transactions. In due course PHARMAC will have full budget management for both these areas of business.

Pharmaceutical Management Agency
Level 9, 40 Mercer Street, PO Box 10-254, Wellington 6143, New Zealand
Phone: 64 4 460 4990 - Fax: 64 4 460 4995 - www.pharmac.govt.nz
Freephone Information line (9am-5pm weekdays) 0800 66 00 50