

Joint submission on PHARMAC OPP Decision Criteria

Chaired by Medicines New Zealand

21 April 2014

We appreciate the opportunity to comment on *PHARMAC's decision Criteria Proposal for change*

We believe that the impact of any change in the decision criteria hinges as much on how they are implemented as what constitutes the criteria.

We have concerns about the proposed framework having the potential to make implementing the decision making less transparent than it is currently unless:

1. Each criterion (factor) is clearly and precisely defined and the definition forms part of the OPP document
2. The subsequent consultations provide a robust process by which the decision criteria are applied and reported on.

Overall commentary

Although the proposal for change appears to be more an attempt to explain what PHARMAC already takes into account than a proposal for substantial change in the aspects it considers, even this has the potential to make the PHARMAC decision making process more accountable, but only if the process issues are addressed in a meaningful way in subsequent consultation.

Currently PHARMAC proposes to have the "supporting information" contained in Appendix 5 of the consultation not form part of the OPP document. We believe this is unworkable as the decision criteria cannot stand alone without being defined. There is already concern that PHARMAC is free to interpret its criteria as it sees fit and inconsistently; and without clear definition of each criterion this would risk the new proposed decision matrix being even more opaque than the current set of criteria. We believe that PHARMAC should be committed to a specific definition of each criterion and that this should only change after consultation in future.

We are concerned that by having the "Supporting Information" as an appendix to the consultation and not considered as a proposed part of the OPP document, submitters will not have adequate opportunity to make submissions on the substance of the "Supporting Information" and whether the concepts contained in it adequately define the decision criteria.

For clarity we will continue to refer to the "factors PHARMAC considers" as "criteria" in this submission and recommend that this terminology is utilised by PHARMAC. To refer to "factors that PHARMAC considers" does not improve the transparency of the decision making process in our view.

We are concerned that this decision criteria consultation does not specifically refer to the separate consultation on access for people with rare disorders and does not appear to have been developed to work alongside the new proposed scheme for high cost medicines. We have not made substantial reference to medicines for rare disorders in this submission however as we will include this in the concurrent "Discussion document" on High Cost Medicines for Rare Disorders.

Proposed definitions of the decision factors

We propose that all of the decision criteria are supplemented by a definition and have tried to provide these definitions. We recognise that our proposed definitions may not reflect the complete and precise meaning attributed to the criteria by PHARMAC, and this is partly why it is so important to provide comprehensive, accurate definitions to form part of the OPP document and have them consulted on. We do not propose that PHARMAC replaces the supporting information which contains more detail, rather we seek to add definitions to clarify the precise meaning attributed to the criteria.

Need

The health need of the patient population under consideration relative to all eligible people in NZ

- The extent of illness (or health deficit) within the group of patients for whom the treatment is being considered. This measure includes how many people are affected as well as how severely they are affected on average and compares this to the expected (normal) life expectancy and quality. Aspects of relative severity of the illness; urgency of need and the degree to which that need is unmet will be measured under this criterion. The expected benefit of the treatment being considered is excluded from this criterion as it is covered in the “benefits of the medicine” criterion.

The principles of the Treaty of Waitangi

- Definition in “Supporting Information” is sufficient.

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

- The expected benefit of providing access to the medicine or medical device that would accrue to a specific population group (geographic, ethnic, demographic or socioeconomic) because of there being a pre-existing worse level of health within that population group.

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

- Whether the existing treatments that are publicly funded for the condition under consideration fully treat the condition for all patients and whether (and to what extent) the new treatment under consideration improves the expected benefit for any of the group of patients.

Supporting Government Health Priorities

- Where a treatment has evidence of being effective in a condition that forms part of the Government’s stated health priorities this would increase the level of priority attributed to funding the treatment under consideration.

Benefits

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

- The measurable improvement in health (measured in QALYs) that the treatment under consideration is expected to provide compared to the treatment which it would replace in clinical use. Where any positive benefit should be balanced against any expected risk attributable to the new treatment.

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

- The measurable overall change in health service utilisation (e.g. hospital admission days; DHB personnel utilisation and other resource use within the health sector) or costs that would be expected to accrue to any part of the health sector if the treatment under consideration is funded.

Costs

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

- The costs that a patient using a treatment would incur due to the use of that treatment, including any overall increase or decrease in such costs to a patient and their family.

Cost of the medicine or medical device

- The cost effectiveness (dollars per unit health gain) of the treatment under consideration, including assumptions of how many people would use it and what existing treatments would be replaced by the new treatment.
- Costs are compared across treatments for different disease groups, patient types, health technologies.
- The timing of the costs is also taken into account, comparing both immediate and future costs and cost offsets (including prevention compared to treatment).
- Note it is unclear whether this criterion is also intended to include the annual budget impact of the treatment under consideration and if so, should include the definition currently under “statutory objective” below.

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

- All costs and savings incurred by any agency funded by Vote Health that would be expected from the decision to make the treatment under consideration available.

Suitability

Suitability of the medicine or medical device to the patient

- The degree to which the attributes of the treatment under consideration are considered beneficial to the patients who would be using it in terms of ease of administration, need for training and overall acceptability of the medicine. Such attributes and benefits will relate only to those with a beneficial impact on health and not simply to stated preferences without a positive benefit to health.

Suitability of the medicine or medical device to the health sector

- The degree to which the attributes of the treatment under consideration are considered beneficial by the prescribers who would be using it in terms of ease of administration, need for training and overall acceptability of the medicine. Such attributes and benefits will relate only to those with a beneficial impact on health and not simply to stated preferences without a positive benefit to health.
- The degree to which the treatment under consideration meets best clinical practice guidelines.

Statutory objective

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

- Would the funding needed to make the treatment under consideration available to the population for whom it is being considered fit within the short term (annual) and long term (3 year) budget?
- The quantity of health gain relative to other investment opportunities is measured in the “Clinical benefits and risks” criteria.
- The definition of “pharmaceuticals” in this context refers to medicines, medical devices, vaccines and related things.

Specific questions in the consultation:

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

- a. If the “high level summary” refers to the three dot points under section 2.1 of the consultation document then the proposed “high level summary” is entirely unhelpful in that it oversimplifies the decision criteria to the point of being almost meaningless.
- b. If however the high level summary refers to the “Supporting information” in appendix 5 of the consultation document then it is helpful but incomplete. We also consider that the factors in the decision matrix are not sufficiently descriptive to stand alone without information that defines their meaning and intent. To this end there is a need for a list of definitions of the factors to form part of the OPP and remain consistent over time.
- c. Any defining information should contain a statement about the degree to which the criteria refer to different health technologies in a similar or different way. I.e. do any differ between medicines, devices and vaccines?

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?

- a. We believe that changing the terminology from “decision criteria” to “factors for consideration” makes no substantial improvement in understanding the decision making process and that the term “decision criteria” is preferable. It is already understood that the criteria are not applied in a tick box manner and the “matrix” further reinforces this message.

3. How would the presentation of a decision-making matrix provide clarity over what PHARMAC considers when it makes a funding decision?

- a. Having a matrix does not in itself add substantial clarity to the decision making, but having definitions of the criteria and reporting on how they are applied on an ongoing basis would be beneficial. We recognise this is a process issue and trust that this will be considered in the next part of the OPP review.

- b. The best mechanism of providing clarity about what PHARMAC actually considers will be to provide ongoing communication of its' application of the criteria in a public summary document for each 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?

- a. There should be different criteria applied at a Board level from a PTAC level as PTAC should not be assessing the decision based on overall budget impact while the Board should. The rest of the criteria should largely be applied consistently in all PHARMAC decisions other than for treatments for rare diseases. If PTAC truly were to take into account the budget impact in their recommendations then PHARMAC should be expected to implement PTAC recommendations with no further consideration or delay.
- b. We believe that there should be a difference in the criteria applied to at a population level and an individual level, particularly in terms of applying a cost-effectiveness criterion. For individual patients it is possible to know more about the severity of the disease and how it is affecting the person in question compared to at a population level.
- c. The decision criteria should not be applied to medical device pilot introduction or evaluation programmes such as medical device "validation" sites in DHBs.

5. Are there other dimensions that you would include? Are there any dimensions that you would leave out? Why?

- a. We are disappointed that PHARMAC continues to avoid referring to community values and ethical frameworks in the decision criteria when it continues to implicitly base decisions on the "utilitarian" framework. It is misleading to state that it would be too difficult to incorporate community values as we recommended in our previous submission that a research survey should be done to guide what is included in community values. A pilot research project has been undertaken by researchers at Otago University and the results show that there are meaningful conclusions to be drawn from such as survey into community preferences.
- b. We strongly support the suitability criterion and point out that for it to be meaningfully implemented there needs to be a corresponding opportunity for patient's perspectives to be provided and considered in the process.
- c. The health opportunity costs of not funding a treatment should be explicitly considered as without such consideration there is a risk of decision makers doing harm from inactivity. I.e. where patients are harmed by a treatment not being funded is as much the responsibility of decision makers as any other aspect of the evidence that is considered.
- d. Equity of health outcomes should be stated either as a criterion or as an interpretation of one of the criteria. E.g. life-saving treatment favoured over minor ailment treatment. The current equity of access is focussed on ensuring that people have equity to a treatment with little attention to the level of health they would achieve using the treatment, whereas if equity of outcomes were used, the focus would be more on providing treatment to those people with more serious illness or a worse state of health to begin with.

- 6. What alternatives to the proposed decision-making matrix could PHARMAC use for presenting what it takes into consideration?**
- The matrix is sufficient as long as other aspects included in our submission are implemented.
- 7. What factors for consideration could be omitted or what further ones could be included to inform PHARMAC's decisions?**
- We believe that the factors referring to cost of the medicine and flow on costs should more explicitly refer to cost effectiveness rather than simply stating "costs" as this is potentially misleading.
- 8. How useful is it to frame the factors for consideration within the broader operating environment that PHARMAC operates within?**
- It is helpful to provide context to the decisions that PHARMAC makes.
- 9. What key strategic or legislative obligations would you omit or include? Why?**
- We recommend that the Wider legal framework includes :
 - The New Zealand Medicines Act 1981 – As this is the legislation that guides the use of medicines in New Zealand.
 - The UN "Convention on the rights of people with disabilities" to which New Zealand is a signatory and which provides substantial guidance about people's rights to treatments.
- 10. What would be achieved by broadening the health disparity factor to include any population groups experiencing health disparities?**
- We consider this to be a meaningful attempt to respond to established need in specifically defined populations or communities. It is one way of improving PHARMAC's responsiveness to communities and targeting health subsidies appropriately.
 - We are however disappointed that it doesn't recognise the currently disadvantaged groups of patients such as those with rare disorders, for which a meaningful solution to optimal access is yet to be established.
- 11. How is the Treaty of Waitangi best reflected in the proposed framework? Why?**
- The Treaty is adequately and appropriately reflected in the framework.
- 12. What would be the impact of removing the current decision criterion 9 ("such other criteria as PHARMAC thinks fit")?**
- We support this criteria being removed and recognise that with adequate consultation there is the opportunity for additional criteria to be included over time if this becomes necessary.

13. What is your view on the proposed rewording of the factors for consideration?

- a. The proposed rewording of the criteria is acceptable ONLY if the supplementary information forms part of the OPP document and there is a commitment from PHARMAC to interpret the criteria in a consistent and transparent manner. We have provided suggested wording of the definitions in part by extracting these from the proposed supplementary information (Appendix 5) and where necessary by proposing entirely new definitions where none were provided in Appendix 5.

In the absence of definitions of the criteria being provided these would be open to a wide range of interpretations and this would not be beneficial to either the clarity or consistency of decision making.

14. Which factors, if any, are unclear or confusing?

- a. All of the criteria need interpretation in a set of definitions as they can have many interpretations depending on the perspective taken. Interpretation should be included in the OPP document and be consistent (committed) over time.
- b. One criterion that is currently problematic even with the definitions provided in the supplementary information is the “cost of the medicine or medical device”. We consider that to accurately reflect what PHARMAC takes into account this should refer to “cost effectiveness” or some other measure of cost relative to therapeutic value.

15. How helpful would the inclusion of a supporting information document be? How would the draft document (appendix 5) provide more clarity and transparency?

- a. We believe that this is an absolutely essential component of the decision criteria as the criteria (or factors) are simplified statements that would be interpreted in many different ways by different people and at different times. In order for the intent of the criteria to be clear and to remain consistent with time there needs to be adequate interpretation as part of the OPP document.
- b. The current supporting information however does not appear to have been developed with this consideration in mind and so this information needs to be redeveloped and ideally consulted on subsequently. We have proposed a set of definitions for the criteria and in the absence of further consultation on these proposed definitions would like to discuss them in detail with PHARMAC prior to them being included in the OPP.

16. What are the pros and cons from using the same decision-making matrix decision criteria for medicines and medical devices? Why?

- a. We consider that comparability between different treatments or technologies is important to ensure that investments are being made in the most cost effective treatment available. This should then result in the most efficient allocation of resource within PHARMAC. There are however differences in the technologies and these differences should be explicitly considered when defining how the criteria relate to specific technologies. For example where Vaccines provide prevention as opposed to treatment this should be taken into account within the clinical benefits and risks of the product.

17. What additional considerations relevant to medical devices could be captured in the proposed decision-making matrix?

- a. We do not hold a position on this.

18. Does the proposed approach reflect your views on ‘community values’? Why or why not?

- a. No. Currently PHARMAC practices an unstated preference for a utilitarian framework of resource allocation and yet PHARMAC says it isn’t possible to include community values.

We believe that it is not only possible, but extremely important for PHARMAC to be guided by the views of the community it serves. Without making an explicit decision on the community values that guide PHARMAC, it will continue to focus more on cost containment than optimal access to treatments for people who need them.

19. What aspects of your ‘community values,’ do you feel are not captured in this proposal?

- a. Equity of health outcomes should be stated either as a factor or as an interpretation of one of the factors. E.g. life-saving treatment favoured over minor ailment treatment. The current equity of access is focussed on ensuring that people have equity to a treatment with little attention to the level of health they would achieve using the treatment, whereas if equity of outcomes were used, the focus would be more on providing treatment to those people with more serious illness or a worse state of health to begin with.
- b. The current model considers opportunity costs only in terms of Dollars, we believe there is an equally strong case to consider opportunity costs in health outcomes. The decision makers should be aware of the health benefits that are not being gained due to the decisions being made. Where there is evidence of disease burden in a population and the Disease adjusted Life Years can be calculated, this estimation should be a factor in deciding the level of priority the associated treatment should be given.

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?

- a. PHARMAC should be prepared to recognise and adequately value innovation in all health technologies it is involved in funding. Where there is a new approach to treating a condition for which there is no equivalent treatment, PHARMAC should be able to include this aspect in the decision criteria so that these treatments are funded with a high priority and made available to patients within a timely manner.

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

- a. The proposed approach does not helpfully address the lack of access to treatments for people with rare disorders. We note however that there is a “Discussion Document” released by PHARMAC for comment to which we will respond with our suggested improvements.

Thank you for the opportunity to submit on this consultation.

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