

Response

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What is your name?

7

Please select the type of individual(s) or organization(s) you represent. Please select all that apply. - Selected Choice

8.1

What is the name of your organisation? - My organisation is called: - Text

9

Are you making feedback on behalf of your organisation?

13

Please select which chapter/s you would like to provide feedback on. You may provide feedback on as many or few chapters as you wish.

1. Transparency, communication, and stakeholder involvement in HTA.3. Methods for HTA for Australian government subsidy (technical methods)

14

Please select the topics within the chapter(s) you would like to provide feedback on. 1. Transparency, communication and stakeholder involvement in HTA

1.1. Transparency and communication of HTA pathways, processes and decisions.1.2. Consumer, clinician and other stakeholder engagement and consideration in HTA

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Please select the topics within the chapter(s) you would like to provide feedback on. 3. Methods for HTA for Australian government subsidy (technical methods)

3.2. Clinical Evaluation Methods.3.3. Economic evaluation

21

Taking all Options within this section: 1.1. Transparency, communication and stakeholder involvement in HTA into account.

Overall, to what extent could the options (if implemented) address the issues that relate to them?

Address some but not most of the issue(s)

23.1

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Publish plain language summaries

Very positive

23.2

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Improvements to the HTA webpage including development of a dashboard

Positive

27

Taking all Options within this section: 1.2. Consumer, clinician and other stakeholder engagement and consideration in HTA into account.

Overall, to what extent could the options (if implemented) address the issues that relate to them?

Address some but not most of the issue(s)

28

If you would like to expand on your answer above you can do so below:

The lived experience and health outcomes of patients with chronic and complex conditions is not adequately understood or appropriately considered under current HTA policies and methods. Implementing the proposed options for section 1.2 may help to rectify this considerable flaw that is leaving some critically ill patients behind.

29.1

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Develop an engagement framework

Very positive

29.2

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Strengthen consumer evidence

Very positive

31

If you would like to expand on your answer above you can do so below -Develop an engagement framework

Involving consumers, clinicians and other relevant stakeholders earlier and more consistently throughout the HTA processes is critical to evening up the playing field for people with complex, uncommon, and heterogeneous diseases who are currently being left behind.

32

If you would like to expand on your answer above you can do so below -Strengthen consumer evidence

This would help consumers to provide more relevant information to inform committees' deliberations.

84

Taking all Options within this section: 3.2. Clinical Evaluation Methods into account.

Overall, to what extent could the options (if implemented) address the issues that relate to them?

Mostly address the issue(s)

85

If you would like to expand on your answer above you can do so below:

Current clinical evaluation methods for HTA - particularly for assessing medicines for inclusion on the PBS - is narrow and rudimentary, focusing on the lowest common denominator and taking a "one-size-fits-all" approach at the expense of people with complex, uncommon and heterogeneous diseases. Clinical evaluation methods need to be updated so that committees can make an informed assessment of the economic costs and benefits of funding health technologies, despite the complexity of diseases that they have been designed to treat.

86.1

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Overarching principles for adopting methods in Australian HTA

Very positive

86.2

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Methods for the assessment of nonrandomised and observational evidence

Very positive

86.3

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Methods for the assessment of surrogate endpoints

Neutral

86.4

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Generate a curated list of methodologies that are preferred by decision-makers, in collaboration with evaluation groups and sponsors.

Very positive

86.5

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Develop an explicit qualitative value framework

Very positive

86.6

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Therapies that target biomarkers (e.g. tumour agnostic cancer therapies, therapies that target particular gene alterations)

Neutral

86.7

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Pharmacogenomic technologies

Neutral

90

If you would like to expand on your answer above you can do so below -Methods for the assessment of nonrandomised and observational evidence

Generating evidence from direct randomised trials is entirely achievable for common conditions which develop and manifest in a typical manner, such as diabetes or asthma, where the clinical benefits of medicines can be easily demonstrated. But for less common and more complex conditions like lupus, which affect each patient differently, it becomes more difficult to demonstrate the clinical effectiveness of medicines, particularly where what works well for one lupus patient might not work for another. Thus, more traditional forms of evidence such as RCT are not suitable. As noted by the Reference Committee, the evidence base for health technologies and methods for assessing evidence are evolving, particularly for rare diseases. As such, nonrandomised and observational evidence should be given greater regard as part of clinical evaluation methods in order to provide patients with complex, uncommon and heterogeneous diseases with reasonable access to more effective treatments.

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If you would like to expand on your answer above you can do so below -Develop an explicit qualitative value framework

This may help to more appropriately and fairly cater for people with complex and less common diseases, which affect each patient differently (like lupus), and thus where traditional clinical evidence (such as RCT) may be unsuitable.

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Taking all Options within this section: 3.3. Economic evaluation into account.

Overall, to what extent could the options (if implemented) address the issues that relate to them?

Address some but not most of the issue(s)

97

If you would like to expand on your answer above you can do so below:

Economic evaluation should take into account the broader economic impacts of improved health function and quality of life for patients who may be able to make a greater economic and community contribution as a result of more effective medical treatment and improved health outcomes. For example, having access to medicines that more effectively treat Systemic Lupus Erythematosus (SLE) would reduce my burden on the health system through fewer doctors visits and hospital stays. It would increase my ability to work more hours, enhance my performance at work, and reduce the amount of sick leave I need to take (and given that Australia has a national skills shortage, supporting people to utilise professional skills to their full potential will help the Australian economy). By slowing or preventing organ damage caused by SLE my economic participation would also be extended over my lifetime. As well as improvements in my own quality of life and health outcomes, the burden of care would be reduced for my partner, which would in turn increase his economic participation.

98.1

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Selection of the comparator

Very positive

98.2

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Valuing of long-term benefits

Positive

98.3

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Valuing overall

Positive

100

If you would like to expand on your answer above you can do so below -Selection of the comparator

This would be particularly helpful for health technologies developed to treat complex, uncommon and heterogeneous conditions. For example, I take multiple prescription medicines that help to minimise the symptoms of Systemic Lupus Erythematosus (SLE), but do not actually treat SLE. PBAC has twice considered a biologic medicine specifically developed to treat SLE (Anifrolumab Saphnelo). Each time it acknowledged the clinical benefits of Saphnelo compared to current SLE treatments, and improvements in disease activity for some patients, but felt that the magnitude of benefit was modest and uncertain. While PBAC acknowledged that the uncertainty was likely impacted by patient heterogeneity and the complex and variable nature of SLE, it nevertheless asked the sponsor to develop a simpler more robust economic model, which is entirely unrealistic. Investigating situations where it may be appropriate to move away from current method/s used in the application of selected comparators might mean that more effective medicines are listed on the PBS, in which case the Government could stop subsidising my consumption of numerous conventional medicines that don't treat my disease and instead pay for a single medicine that does.

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In summary, considering all the draft reform options together:

How confident are you that the reform options (if implemented) will make health technology assessments better overall?

Somewhat confident