

Response

2

The Department may, at its discretion, publish part or all of the information provided in your submission on the Department's website or in related documents. If information from your submission is published, the Department may identify you and/or your organisation as the author of the submission. All personal contact details will be removed prior to publishing.

Yes, I consent to my identified submission being published

3

What is your name?

Hania Hussain

7

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

Patient or consumer (or representative organisation)

8.1

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

Mito Foundation

9

Are you making feedback on behalf of your organisation?

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,2. Health technology funding and assessment pathways,3. Methods for HTA for Australian government subsidy (technical methods),4. Health technology funding and purchasing approaches and managing uncertainty,5. Futureproofing Australia's systems and processes

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,1.3. First Nations people involvement and consideration in HTA,1.4. State and territory government collaboration in HTA

15

Please select the topics within the chapter(s) you would like to provide feedback on. 2. Health technology funding and assessment pathways

2.1. Streamlining and aligning HTA pathways and advisory committees,2.2. Proportionate appraisal pathways

16

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)

3.1. Determination of the Population, Intervention, Comparator, Outcome,3.2. Clinical Evaluation Methods,3.3. Economic evaluation

17

Please select the topics within the chapter(s) you would like to provide feedback on. 4. Health Technology funding and purchasing mechanisms and decisions

4.1. Approaches to funding or purchasing new health technologies,4.3. Understanding the performance of health technologies in practice

18

Please select the topics within the chapter(s) you would like to provide feedback on. 5. Futureproofing our systems and processes

5.1. Proactively addressing areas of unmet clinical need and gaps in the PBS,5.2. Establishment of horizon scanning programs to address specific informational needs within HTA and the health system

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)

22

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

The proposed measures are essential to the HTA reform process, and their implementation should be prioritised. It is also important that these changes are not made as standalone measures, as they are insufficient to address the broader needs of consumers and other stakeholders on their own. They should be part of the broader reform effort, incorporating suggestions outlined in the Options paper to ensure a more inclusive, efficient and patient-centred HTA framework. This is also in line with the guiding principles laid out in the National Medicines Policy.

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

Very positive

26

If you would like to expand on your answer above you can do so below -improvements to the HTA webpage including development of a dashboard

Key Performance Indicators should be included in the dashboard. This should include both the target and the actual performance against these indicators.

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?

Mostly address the issue(s)

28

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

Consumer, clinician and other stakeholder engagement and consideration in the HTA should be integral to the HTA processes. This is especially important for rare and ultra-rare diseases: they have limited awareness and experts in the general population and members of HTA committees may lack this expertise. It is, therefore, essential to recognise the expertise of consumers and their clinicians in the HTA processes.

The proposed reform options, if implemented properly, are a step in the right direction. Given that the consumer engagement co-design process is still ongoing, fully assessing the potential impact of these reforms poses a challenge. Nevertheless, we think the process would benefit from increased transparency around:

- the criteria for proposed horizon scanning and the definition of 'æhigh unmet clinical need', especially for rare/ultra-rare diseases,
- submission outcomes, including clarity on commercial-in-confidence issues, which often present significant barriers to transparency.

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

It is important that this framework is co-designed with stakeholders to ensure successful design and implementation. Successful implementation requires clarity regarding responsibility for the engagement work. If patient organisations are expected to undertake additional engagement efforts, it's imperative to address how this work will be resourced. Given the diverse capabilities and capacities within the patient organisation sector, there is a risk that insufficient resources may impede the necessary engagement, potentially resulting in some applications lacking the consumer evidence required for information decisions. Resourcing was also highlighted as a critical consideration in the Conversations for Change report.

Moreover, the Department likely has a role to play in prioritising or triaging applications based on their need for engagement and consumer evidence, ensuring that resources are allocated effectively to maximise impact and outcomes.

32

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

It's crucial to engage consumers and truly incorporate their perspectives across all decision-making processes, including adopting a broader definition of value and utilising patient-reported outcome measures and preferences. This is especially important for people living with rare diseases, where opportunities to participate in clinical studies are limited, and effective therapies are scarce. Better clarity is also needed on how smaller patient organisations/ individual consumer will be supported, and the resources required to provide this support.

39

Taking all Options within this section: 1.4. State and territory government collaboration 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)

40

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

This option seems positive in that it stresses the importance of streamlined collaboration between the state and territory governments to ensure efficient decision-making processes. The proposed changes could potentially yield positive outcomes by improving timeliness, efficiency, and responsiveness within HTA processes. However, the options paper is unclear on how this will be implemented. More details are essential to ensure that the intended benefits are realised without compromising the effectiveness of the HTA procedures.

Timely and equitable access to health technologies are major priorities, and the reform should ensure that these should be at the forefront of all considerations and changes.

41.1

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Development of central standardised data sharing system for utilisation and outcome data

Positive

41.2

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Increase opportunities for consultation and work sharing

Don't know

41.3

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Health technologies that are jointly funded by the Commonwealth and state and territory governments (such as high cost, Highly Specialised Therapies (HSTs) delivered to public hospital inpatients)

Positive

46

Taking all Options within this section: 2.1. Streamlining and aligning HTA pathways and advisory committees into account.

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

Don't know

47

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

Mito Foundation supports the measures described in these reform options that have the potential to increase timely and equitable access to health technologies for all Australians. However, an implementation plan is needed which takes the following into account:

- Consumers are engaged throughout this process. All decisions should be made with appropriate expertise guiding decision-making. This includes rare disease expertise and consumer representatives.
- Strengths in existing processes are not lost; whatever changes are made still need to be fit for purpose. One example is the expertise of MSAC in making decisions about genomic testing.
- Clarity on the transition process of existing committee(s) functions to the PBAC and then to the HTA committee.
- Any changes should ensure that there are no additional barriers to access for complex, high-cost treatments for ultra-rare diseases.
- Ensure that the streamlined pathway doesn't instead add inefficiency to the HTA system due to the increased scope of health technologies.

48.1

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Pathway for drugs for ultra-rare diseases (Life Saving Drugs Program (LSDP))

Positive

48.3

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

Positive

48.4

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Unified HTA pathway for all health technologies with Commonwealth funding

Positive

50

Pathway for drugs for ultra-rare diseases (Life Saving Drugs Program (LSDP))

Any decisions made should be guided by appropriate expertise, including rare disease experts and consumer representatives. The inclusion of LSDP decisions into PBAC has the potential to shorten the time taken to make a decision. However, decisions must continue to be based on a distinct set of guidelines and ensure that appropriate rare disease clinical and consumer expertise.

Any change made should ensure that there are no additional barriers to access for complex, high-cost treatments for ultra-rare conditions.

61

If you would like to expand on your answer above you can do so below -Expanding role of PBAC

In transitioning to the PBAC, the HTA should not lose the current fit-for-purpose functions of other committees such as MSAC. All changes should ensure that we are introducing efficiency to the process without losing capabilities and flexibility.

62

If you would like to expand on your answer above you can do so below -Unified HTA pathway for all health technologies with Commonwealth funding

While the idea of a single HTA body is appealing, we are concerned that the Options paper does not provide much clarity on how the unified body would incorporate expertise for such a large range of health technologies. The existing pathways have some merit; these need to be appreciated and incorporated into the development of the new HTA body.

63

Taking all Options within this section: 2.2. Proportionate appraisal pathways into account

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

Don't know

65.1

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

Positive

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

Positive

65.9

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Decouple the requirement for the TGA Delegate's overview to support PBAC advice

Very positive

67

If you would like to expand on your answer above you can do so below -Triaging submissions

Triaging of submissions can streamline the HTA appraisal processes by making sure that we spend the right amount of effort for each submission. However, this process should be transparent and co-designed with consumers. The framework should explicitly define HUCN, 'aerisk and other factors', and prioritise timely and equitable access to the HTA, particularly for rare diseases.

75

If you would like to expand on your answer above you can do so below -Decouple the requirement for the TGA Delegate's overview to support PBAC advice

Parallel processing holds a lot of promise. The current two-stage process continues to add unnecessary delays to access in Australia.

76

If you would like to expand on your answer above you can do so below -Case manager

Depends on the definition of their role and their expertise. Could potentially be positive but could also reduce efficiency.

77

Taking all Options within this section: 3.1. Determination of the Population, Intervention, Comparator, Outcome into account.

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

Mostly address the issue(s)

78

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

Early consultation on the PICO is a key opportunity that can incorporate consumer input. This includes in understanding the population, confirming relevant comparators and providing valuable real-world evidence of outcomes that matter to patients. This would be an ideal topic where guidance could be provided to consumers and consumer organisations to assist them in providing useful input.

Consultation on the PICO is a strength of the current MSAC process.

79.1