

Response

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Yes, I consent to my identified submission being published

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What is your name?

Kristina Elvidge

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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

Childhood Dementia Initiative

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Are you making feedback on behalf of your organisation?

Your organisation

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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

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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,1.4. 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

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

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,5.4. Mechanisms for continuous review and improvement,5.5. Capacity and capability of the HTA system,5.6. Strengthen international partnerships and work-sharing

21

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

22

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

Mostly address the issue(s)

22

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

The options demonstrate the voice of the consumer has been heard in the HTA review. If progressed these options will provide a fundamental platform for engagement to embed the consumer as a valued and equal contributor to the HTA process.

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

27

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

28

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:

Resources need to be made available for consumer input, both training, and reimbursement for time. Not all patient organisations or individual consumers have the capacity to do this important work without funding being made available. This will ensure that input is equitable.

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

39

Taking all Options within this section: 1.4. State and territory government collaboration in HTA into account.

40

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

Mostly address the issue(s)

40

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

This option is critical to improve equity in access to medicines. Subsidy policies vary between states and cause inequity and confusion. To be a leader in adoption of this new technology implementation of this collaboration is very important.

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

Very 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

Very positive

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)

Very positive

45

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

Many costly gene therapies are coming down the pipeline for rare genetic diseases like childhood dementia disorders, so funding models that share the cost and make them equitably available to all patients across the country are essential.

46

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

47

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

Mostly address the issue(s)

47

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

Streamlining HTA processes is welcomed to reduce the time from TGA registration to listing on the PBS. We hope that this major change will not slow down processes in the meantime and it can be implemented in a timely manner without too much disruption to getting life saving medicines to patients.

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))

Very positive

48.2

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

Neutral

48.3

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

Don't know

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

Very positive

50

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

We support a single assessment process for ultra rare disease therapeutics to speed up access, however it is important to retain existing guidelines and clinical expertise that is a strength of the existing LSDP process.

77

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

78

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:

Clarity on the PICO criteria is needed, while not creating further delays. Early engagement with relevant consumer groups and expert clinicians, particularly as a result of horizon scanning is a key way that this can be accomplished.

79.1

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

Very positive

79.2

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

Very positive

79.3

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

Very positive

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:

We agree that HTA assessments should allow more flexibility in the evidence base, and greater acceptance of non-randomised evidence, the role of RWD and surrogate outcome measures. The inclusion of consumer evidence and RWE evidence will be fundamental to deliver access to therapies for rare conditions and for underrepresented populations.

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

Very positive

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.

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

Positive