

Response

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Yes, I consent to my identified submission being published

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What is your name?
Louise Healy

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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
Rare Voices Australia

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

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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,1.3. 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

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

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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,4.2. Approaches to incentivise development of products that address antimicrobial resistance (AMR),4.3. Understanding the performance of health technologies in practice

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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,5.3. Consideration of environmental impacts in the HTA,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.
Overall, to what extent could the options (if implemented) address the issues that relate to them?
Mostly address 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
25
If you would like to expand on your answer above you can do so below -Publish plain language summaries
We support these summaries as essential for informed consumer input, consideration needs to be given to how the summaries will be developed, and who will develop them, to ensure that they provide necessary information in an independent and fully transparent way.
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
Performance statistics are important inclusions and should be developed using the objectives of the NMP.
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)
29.1
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Develop an engagement framework
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 essential that this framework is codesigned with stakeholders in order to achieve its objectives. This framework should also consider engagement mechanisms for priority populations, including people from culturally and linguistically diverse (CALD) communities and those with disabilities.
32
If you would like to expand on your answer above you can do so below -Strengthen consumer evidence
It is unclear what role the existing Consumer Evidence and Engagement Unit will play here, but it is important from a rare disease perspective that there is a mechanism to support involvement from small consumer groups/organisations with limited resources, or individual consumers from very small patient populations where no consumer group/organisation exists. The existing Consumer Evidence and Engagement Unit is well placed to take on this role.
33
Taking all Options within this section: 1.3. First Nations people involvement and consideration in HTA into account.
Overall, to what extent could the options (if implemented) address the issues that relate to them?
Don't know
34
If you would like to expand on your answer above you can do so below:
We support all implementation mechanism that support improved outcomes for First Nations people, noting that those with rare diseases face additional inequities that may need to be prioritised through measure outlined to address areas of high unmet clinical need.
35.1
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - First Nations peoples partnership in decision making
Don't know
35.2
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Dedicated resource for HTA submissions and education
Don't know
38
If you would like to expand on your answer above you can do so below -Dedicated resource for HTA submissions and education
We believe a central resource is the most sustainable and equitable approach.
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)
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
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)
Don't know
43
If you would like to expand on your answer above you can do so below -Development of central standardised data sharing system for utilisation and outcome data
Strongly support this but believe it should align with other initiatives (e.g clinical quality registries) to reduce duplication and leverage existing funding.
44
If you would like to expand on your answer above you can do so below -Increase opportunities for consultation and work sharing
Strongly support this as a mechanism to ensure consistent and equitable access across Australia. Consistent timeframes for implementing positive recommendations from committees should be agreed and be part of KPIs. E.g. a medicine will be available to consumers within 6 months of a PBAC recommendation being accepted by government or a test recommended by MSAC will be available within 6 months if infrastructure equipment already exists (e.g. MRI for a rare indication) or 12 months if procurement of equipment or expertise is required.
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)
Addresses technologies that are jointly funded but does not discuss or identify funding pathways for those technologies that may not fit current funding pathways. For example the acute care genomics project that provided rapid trio WGS for infants in hospital and their parents currently has no clear funding pathways in current HTA/NHRA processes. This is because the child requiring testing is an admitted patient in a public hospital and consequently, is not eligible for Medicare Benefits Scheme (MBS) funding. Similarly, the parents who also require testing are not eligible for hospital funding as they are not admitted patients. These processes have already created inequities with some states (Victoria and Western Australia) providing specific funding for this program, while babies in other states are not receiving this testing.
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?

Address some but not most of the issue(s)

47

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

We support measures to reduced delays and increases timeliness and equity in access for patients. All consideration of technologies for rare diseases must be informed by appropriate expertise, including consumer expertise.

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))

Neutral

48.2

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

Don't know

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

Neutral

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

Any changes to assessment of technologies for ultra-rare diseases must be in line with current guidelines and with appropriate expertise guiding decision making - this would include rare disease expertise and consumer representatives. Any change 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

We support measures that will improve timeliness and equity of access to complex or highly specialised health technologies. An expanded role will require PBAC to adopt assessment and processes for managing uncertainty and complexity of technologies for rare/ultra-rare conditions and assessing value for money currently applied in the LSDP process. Assessment processes must be fit for purpose for HUCN/HATV technologies, including health technologies for rare diseases that do not meet LSDP criteria (technologies for rare diseases currently face challenges in meeting the cost effective criteria currently applied by PBAC).

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

RVA supports a unified HTA pathway provided that measures are in place to ensure that the objectives of the NMP are being assessed and that measures improve timeliness and equity of access, while supporting innovation. A unified pathway may also enable the standardisation of timeframes for committees (i.e Medical Services Advisory Committee (MSAC) decisions having standardised timeframes similar to the PBAC). It is unclear from the Options Paper how a unified pathway would apply expertise and knowledge of existing committees, while also including additional expertise such as consumer expertise, where necessary. Such detail is critical.

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?

Mostly address the issue(s)

64

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

Options for more timely and equitable access, particularly for rare disease health technologies where there is often very high unmet clinical need and high-cost technologies, should be prioritised. Triaging systems should reflect the objectives of the NMP and explicitly consider equity, HUCN and innovation. Triaging processes must be transparent and codesigned with stakeholders.