

Response

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Yes, I consent to my identified submission being published

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What is your name?
Gethin Thomas

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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
MND 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), 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

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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

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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, 5.6. 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
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.

Overall, to what extent could the options (if implemented) address the issues that relate to them?
Completely 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
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
This is vital, especially for diseases such as MND where survival is short and it is 100% terminal. Potential impact on patients and families needs to be heard beyond just QoL measures and financial cases. Support should be available to resource smaller consumer representative organisations to provide this engagement and truly represent their consumers.

32
If you would like to expand on your answer above you can do so below -Strengthen consumer evidence
It is critical that there is engagement with consumer organisations when developing criteria for consumer evidence. Every disease will have different criteria that should be considered and there should be scope to tailor evidence according to the disease being considered. A one-size-fits-all approach is not acceptable.

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?
Mostly address the issue(s)
40
If you would like to expand on your answer above you can do so below:
It is critical that patients are not denied cutting edge treatment due to the location.
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

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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?
Mostly address the issue(s)
47
If you would like to expand on your answer above you can do so below:
We need clear clarification about which diseases will be included in the ultra-rare disease category and what the criteria are. There may be genetic forms of a more common disease where this can be less clear e.g. there are genetic forms of MND which are quite rare and specific, but likely expensive, treatments are being developed. Such subsets of diseases could be considered as ultra-rare.
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

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:
A clear definition of which conditions qualify for high unmet clinical need (HUCN) must be provided. Significant consultation with consumers must be undertaken in the consideration of this definition and it should be revisited regularly.
65.1
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Case manager
Very positive
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Triaging submissions
Very positive
65.2
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Streamlined pathway for cost-minimisation submissions (therapies not claiming a significant improvement in health outcomes or reduction in toxicity)
Positive
65.3
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN:

Alternative option 1: Introducing an optional resolution step before HTA committee consideration
Neutral
65.4
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN:

Alternative option 2: Introducing an optional resolution step before HTA committee consideration, with additional post committee resolution
Very positive
65.5

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN:

Alternative option 3: Early Price negotiation

Negative

65.6

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN:

Alternative option 4: Introducing an optional resolution step after HTA committee consideration but before advice is finalised

Negative

65.7

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Expanding resolution step to all relevant cost effectiveness submissions

Don't know

65.8

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Development of a disease specific common model (reference case) for disease areas with high active product development

Very 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

74

If you would like to expand on your answer above you can do so below -Development of a disease specific common model (reference case) for disease areas with high active product development

These need to be generated with strong consumer engagement

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:

The explicit consideration of health equity and priority populations for new treatments needs to be developed with strong consumer consultation. It is critical to ensure diseases such as MND are considered in their own context and not suffer in comparison to other diseases and inappropriate metrics

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

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