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?
Edward Giles
7
Please select the type of individual(s) or organisation(s) you represent. Please select all that apply. - Selected Choice
Clinician (or representative organisation)
8.1
What is the name of your organisation? - My organisation is called: - Text
Monash Children's Hospital
9
Are you making feedback on behalf or your organisation?
Yourself
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)
14
Please select the topics within the chapter(s) you would like to provide feedback on. 1. Transparency, communication and stakeholder involvement in HTA
1.2. Consumer, clinician and other stakeholder engagement and consideration 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
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:
These need to include the needs of the paediatric population, with facilities for their voice to be heard
29.1
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Develop an engagement framework
Neutral
29.2
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Strengthen consumer evidence
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?
Mostly address the issue(s)
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
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))
While IBD, and even paediatric IBD, is not ultra-rare, the subgroup of very young children with IBD (Very Early Onset '“ VEO-IBD) are a very small group with high morbidity, and some mortality. These patients currently lack access to any of the advanced therapies with no clear pathway of access. GESA believes that VEO-IBD, and paediatric IBD should have access to medications, including escalated dosing, through this pathway.
61
If you would like to expand on your answer above you can do so below -Expanding role of PBAC
Due to the current system, the small but significant number of children with inflammatory bowel disease have even less access to medications than adults, as the current processes demand a level of trial data that will never be seen in paediatrics. It would be critical for PBAC to be specifically open to a different process of evidence assessment for children with diseases where treatments are available in adults, but trials have yet to be (or may never be) performed in kids.
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)
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
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
Very positive
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
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
Positive
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
Neutral
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

Neutral

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

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)

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)

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

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

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.

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

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

It is critical with children in particular to use real world evidence and extrapolation - currently Australian children with IBD and being left behind compared to other countries including NZ and Canada.

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