Response
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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?
Trent Zimmerman
7
Please select the type of individual(s) or organisation(s) you represent. Please select all that apply. - Selected Choice
Pharmaceutical / Medical technology company
8.1
What is the name of your organisation? - My organisation is called: - Text
Alexion, AstraZeneca Rare Disease, Australasia
9
Are you making feedback on behalf or 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,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
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?
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
Positive
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?
Completely address the issue(s)
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
Very positive
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
Very positive
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)
Neutral
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)
Implementation plans should be adopted for high-cost specialised therapies that are delivered in an inpatient setting. However, these should cover all therapies and not just CAR-T therapies. The next NHRA should consider funding arrangements with an increase in federal contribution if new inpatient therapies are replacing those that would have previously been delivered through the PBS.
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)
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
48.2
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
Negative
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))

LSDP submissions are intrinsically for therapies where cost effectiveness will not meet normal PBAC requirements. The options paper implies cost effectiveness would be considered by PBAC, which would undermine the very rationale of the LSDP.

Pathways to the LSDP should be determined at initial gateway through triaging following a request from sponsor.

Sponsors should be able to request a stakeholder meeting with an expert panel that includes patients and clinicians based on Scottish PACE model.

Fundamentally, LSDP guidelines should be broadened to include severe morbidity recognising that some ultra rare diseases may not be life threatening but can profoundly affect quality of life. This would significantly increase the benefits of the LSDP for ultra rare disease patients.

The LSDP expert panel should continue to be the primary source of advice on the clinical effectiveness of ultra rare therapies but strongly supports the streamlined pathway to remove the current requirement that LSDP funded therapies are only considered after a PBAC rejection.

Current structure of advice on LSDP to Minister including consultation with the Chief Medical Officer should be retained.

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

Alexion supports a unified pathway for health technology assessments to streamline the process subject to our comments about the LSDP.

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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?

Address some but not most of the issue(s)

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

Neutral

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)

Very negative

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

Negative

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

Negative

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

Positive

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

Positive

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 negative

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

Positive

67

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

In addition to steps in option 4, Alexion recommends that sponsors be able to request facilitated workshops with the PBAC prior to PBAC consideration. This is possible under the current system but only after a negative recommendation and at the request of the PBAC itself. Allowing sponsors to request a workshop and its timing early in the process would considerably reduce time to access and allow the early resolution of issues.

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