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?
Kym Bramich
7
Please select the type of individual(s) or organisation(s) you represent. Please select all that apply. - Selected Choice
Other [please specify]
7.22
Please select the type of individual(s) or organisation(s) you represent. Please select all that apply. - Other [please specify] - Text
Not For Profit membership organisation, structured as a company limited by guarantee
8.1
What is the name of your organisation? - My organisation is called: - Text
Omico
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
1.4. 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)
3.2. 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
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)
40
If you would like to expand on your answer above you can do so below:
Complex health technologies tend to be deployed in the hospital system funded through state/territory mechanisms. State based variations in what is considered to be '˜standard of care' technology leads to incongruous investment in technologies that can lead to unwarranted variation and inequity in access to best practice care. For example, whilst progress has been made, the fact remains that at present, advances in genomics and precision oncology are not being integrated into routine healthcare. Omico supports the creation of a dedicated federal funding mechanism for health technologies which fall into this category, which is distinct from current funding for state healthcare. We bear in mind that federal-state negotiations have historically been complex, so consideration of mechanisms which minimize the dependency of the funding deployment on a state-by-state approval. National infrastructures such as Omico that enable nationally consistent investment into complex health technologies may represent a generalizable solution to this challenge
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)
See above: Federal-state negotiations have historically been complex, so consideration should be given to mechanisms which minimize the dependency of the funding deployment on a state-by-state approval basis. National infrastructures such as Omico that enable nationally consistent investment into complex health technologies may represent a generalizable solution to this challenge.
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:

The major challenge that appears not to be addressed by the review is the co-dependency of therapeutic approvals on companion diagnostic tests.

For cancer, this is a critical issue which must specifically be addressed.

Targeted therapeutics which have been FDA approved in a tumor agnostic fashion represent HATV. In particular, these therapeutics have the potential to address the challenges faced by patients with rare and less common cancers, or cancers of unknown primary site (who clearly represent HUCN patients).

To access such therapies, biomarkers must be identified to ensure maximum patient benefit for cost. Such biomarkers are typically genomic in origin (at least in relation to the current FDA-approved cancer agnostic therapies). To usefully deploy these cancer agnostic therapies, access to molecular screening is an essential requirement for these patient populations. The current HTA pathways gives rise to a failure to reimburse agents such as PDL1 targeting agents, or NTRK inhibitors, in populations who would clearly benefit from access, purely for lack of reimbursed access to genomic and molecular screening.

This problem will become more extreme as an increasing range of such precision oncology therapies is identified, in turn exacerbating the lack of equitable access to targeted therapies for HUCN populations such as the 25,000 Australians estimated to die in 2023 from rare and less common cancers, or from cancers of unknown primary site.

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?

Address some but not most of 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

Don't know

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

Don't know

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.

Neutral

86.5

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Develop an explicit qualitative value framework

Neutral

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)

Positive

86.7

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

Neutral

89

If you would like to expand on your answer above you can do so below -Overarching principles for adopting methods in Australian HTA

The issue of co-dependent technology assessment is critical.
The expanded role of PBAC to take into account co-dependent technologies to identify populations who would benefit from access to HATV is critical to any cancer agnostic pathways.

94

Therapies that target biomarkers (e.g. tumour agnostic cancer therapies, therapies that target particular gene alterations)

Co-dependency of therapeutic approvals on companion diagnostic tests is a critical issue which must specifically be addressed.

Targeted therapeutics which have been FDA approved in a tumor agnostic fashion represent HATV. In particular, these therapeutics have the potential to address the challenges faced by patients with rare cancers, or cancers of unknown primary site (who clearly represent HUCN patients).

However, to access such therapies, biomarkers must be identified that ensure maximum benefit for cost. Such biomarkers are typically genomic in origin (at least in relation to the current FDA-approved cancer agnostic therapies). To usefully deploy these cancer agnostic therapies, access to molecular screening is an essential requirement for these patient populations.

The current HTA pathway gives rise to a failure to reimburse agents such as PDL1 targeting agents, or NTRK inhibitors, in populations who would clearly benefit from access, purely for lack of reimbursed access to genomic and molecular screening.

This problem will become more extreme as an increasing range of such therapies is identified, in turn exacerbating the lack of equitable access to targeted therapies for HUCN populations such as the 25,000 Australians who will die in 2023 from rare cancers, or from cancers of unknown primary site.

103

Taking all Options within this section: 4.1. Approaches to funding or purchasing new health technologies 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)

105.6

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Approaches for managing uncertainty - bridging funding coverage for earlier access to therapies of likely HATV and HUCN

Neutral

105.7

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Approaches for managing uncertainty - revised guidance on the uses of different managed entry tools

Positive

112

If you would like to expand on your answer above you can do so below -Approaches for managing uncertainty - bridging funding coverage for earlier access to therapies of likely HATV and HUCN

The theme of tolerance of uncertainty, or the balance of uncertainty, against unmet need is going to increase as therapies focus on increasingly precisely defined populations of smaller size.

Gathering evidence beyond phase 3 randomised clinical trial (RCT) data is crucial for expediting access to precision oncology therapies for cancer patients, especially those with HUCN and in smaller patient populations (in which RCT data is unlikely to be generated at all or in a very delayed timeframe given small patient numbers). Such evidence, including real-world data, observational studies, and biomarker analyses, can offer insights into treatment effectiveness and safety in diverse patient populations, providing a more comprehensive understanding of therapeutic benefits and enabling faster regulatory approvals and faster patient access.

The typical scenario is an FDA approval of a new HATV in a population with HUCN, without randomized data, or without the prospect of generating such data. Even when data is/can be generated, access to therapies with clearly greater clinical benefit than current standard of care is typically delayed years.

We believe it is worth considering a shared risk model with industry under an expanded managed access program (MAP) with data generation. This could expedite access to such therapies, while at the same time generating the evidence required for unconditional approval.

(See Further Comments section below for continuation of this response.)

121

Taking all Options within this section: 4.3. Understanding the performance of health technologies in practice 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)

125

If you would like to expand on your answer above you can do so below -Oversight " reforms to optimise access to and use of RWD in HTA

It is critical to create national capacity for RWD generation in the service of HTA for use by MSAC and PBAC. Such capacity should ideally be resourced by both government and industry as a vehicle to expedite access to HATV, especially for HUCN patients, while resolving uncertainties created by evidence gaps in relation to the HATV. As much as possible, such national capabilities ought to harness existing infrastructure and data sources in the healthcare system.

127

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

Omico is an example of a not-for-profit, national infrastructure for biomarker-dependent drug development, which collects long term health outcome data on patients with cancer who are treated with HATV. It incorporates the complex technologies which enable identification of populations with HUCN who would benefit from access to HATV, as well as the ability to collect both patient-centred outcomes and health system resource utilisation.

131

Taking all Options within this section: 5.1. Proactively addressing areas of unmet clinical need and gaps in the PBS into account.

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

Address little or none of the issue(s)

132

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

The advent of a molecular view of cancer has enabled development of HATV which target increasingly precisely defined populations, typically using genomic or other molecular screening. In Australia, access to the infrastructure for molecular profiling is limited - this essential diagnostic technology is not available as standard of care.

Access to standard of care molecular profiling will be critical to both the clinical trials competitiveness of the Australian healthcare system, and to the systematic identification of patients with HUCN who would benefit from access to relevant targeted therapies.

Two populations have the greatest potential benefit from the new era of rational drug development: patients with rare cancers (~25,000 deaths/yr in Australia) and patients with cancers of unknown primary site (~2,000 deaths/yr), for whom cancer agnostic therapies based on carriage of a specific biomarker represent a game-changer.

Ultimately, Australia needs to urgently develop a solution to the approval of cancer agnostic therapies. This can be achieved through provision of universal access to molecular profiling, expansion of clinical trials access (including MAPs under the oversight of PBAC), and a high-cost drugs fund which may be linked to industry investment in molecular profiling and expanded clinical trials activity. This approach would require a mechanism for greater engagement between industry and governments, and the ability to measure contributions of both.

167

In summary, considering all the draft reform options together:

How confident are you that the reform options (if implemented) will make health technology assessments better overall?

Not at all confident

241

Do you have further comments or concerns to add specific to this topic that should be considered? For example, here you can detail any unintended consequences or overlooked considerations if applicable.

Continuation of above response in Approaches for managing uncertainty - bridging funding coverage for earlier access to therapies of likely HATV and HUCN:

Such MAPs balance the need to have greater confidence that public reimbursement is justifiable for patients with HUCN to have access to important HATV.

In effect, we advocate for expansion of this capability through a budget for co-investment in MAPs, harnessing national infrastructure to enable equitable access under controlled conditions, and the capability of PBAC to negotiate with the sponsor, the data required for unconditional approval.

The latter would provide certainty for future investment of industry sponsors in data generation, while ensuring resolution of uncertainty on the part of regulatory bodies.