HTA STAKEHOLDER ENGAGEMENT ROUND 2

Full Report V3

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Contents

List of Tables

About this report

Background

The Commonwealth as represented by the Department of Health and Aged Care (the Department) is supporting and resourcing a Health Technology Assessment (HTA) Policy and Methods Review (HTA Review). Health technology assessments inform Australian Government decisions to fund and subsidise health technologies through subsidy schemes and funding programs like the Pharmaceutical Benefits Scheme (PBS) and Medicare.

An HTA involves a range of processes and mechanisms that use scientific evidence to assess the quality, safety, efficacy, effectiveness and cost-effectiveness of health technologies. HTAs help decision makers understand how effective and safe a health technology would be compared with available alternatives, and whether any additional cost is worth paying for.

The HTA Review commenced in March 2023. The HTA Review is being led by a Reference Committee which will prepare a final report with recommendations for reform to government by 4 May 2024.

HTA Options Paper

The Department publicly released a HTA Options Paper on 25 January 2024 on the HTA consultation hub, inviting stakeholders to engage and provide comment in response to the potential options for reform both generally, and more specifically in relation to:

- whether the proposed option/s will achieve the intended outcome,
- what the potential impact on stakeholders may be, and
- any unintended outcomes or challenges stemming from the proposed options.

HTA Consultation Hub

The Consultation Hub offered stakeholders the opportunity to provide feedback through multiple channels:

- by registering interest to attend an online or face-to-face workshop,
- submitting their feedback through an online questionnaire, and/or
- submitting feedback by email to the HTA secretariat.

Consultation workshops

Four (4) facilitated workshops were conducted with stakeholders, three (3) online between the 13th to 16th of February 2024, and one (1) face-to-face workshop in Sydney on 19th of February 2024. The online workshops were three (3) hours in duration and the face-to-face workshop was five (5) hours. A summary of the workshop outcomes is included in Appendix A of this report.

Written submissions

The Department invited written submissions from individuals and organisations to gather feedback on the potential options included in the Options Paper.

In addition to providing opportunities for feedback on individual potential options, the questionnaire enabled stakeholders to upload supplementary information in commonly used file formats (e.g. a Microsoft Word document or an Adobe PDF document). The HTA Review Secretariat also received many submissions via email, which were forwarded to Bastion for inclusion in our analysis and reporting.

This report provides a high-level summary of the one hundred and thirty-two (132) written submissions received from stakeholders across a range of patient advocates, clinicians, industry, researchers, state government officials, consultants and evaluators (see Appendix B for further detail on contributing stakeholders).

Executive summary

There was a multitude of written submissions to the HTA Committee Review, and there is widespread thanks and support for the Committee's extensive consultation and review process. There is wide acknowledgement that the options proposed seek to make the HTA more flexible and efficient to allow timely access to medicines and health technologies for all Australians.

Key views emerging across the written submission process are summarised by reform topics as put forward in the HTA Reform Options Paper below.

Section 1: Transparency, communication and stakeholder engagement

There was strong support and encouragement across the majority of submissions for increased transparency, communication and consultation between stakeholders and the HTA, as well as State and Federal governments. Several written submissions also highlighted that a number of these recommendations could be implemented relatively quickly for immediate impact. It was also recommended by a large group of consumer representatives that the recognition of the value of the consumer voice be formalised through legislation, to ensure that it is safeguarded as part of the HTA. This group also highlighted that if the options in 1.1 to 1.3 in the Options Paper were progressed, they would provide a fundamental platform for engagement to embed the consumer as a valued and equal contributor to the HTA process.

Across the stakeholder groups there was comment on the value that plain language summaries offered in assisting those with lower health literacy to understand the HTA process and the decisions ultimately made. Stakeholders also welcomed improvements and upgrades to the website and stated the introduction of a dashboard to the website would be a valuable new inclusion, especially in terms of increasing accountability on the progress of individual submissions.

There was consensus amongst stakeholder groups that the options in 1.2 in the Options Paper were a step in the right direction for the HTA process, and that increased involvement of stakeholders such as clinicians and consumers would lead to better outcomes for patients.

The vast majority of stakeholder groups see the potential that increased involvement and consideration could have to improve health outcomes for First Nations people. The criticality of supporting all initiatives that contribute to closing the gap and reducing the health inequities for First Nations people were emphasised and noted by all stakeholder groups. The need for KPIs to measure progress on these initiatives was also raised.

There was also a stated belief that increased and formal collaboration with State and Territory governments outlined in 1.4 in the Options Paper would assist with managing funding pathways and allowed for better information sharing, and the potential for a centralised data sharing system was supported broadly. The variations across States and Territories, as well as the need to negotiate to individually with these jurisdictions, was raised as a concern that is not directly solved though these options. The need for a federally funded system for those therapies out of scope for PBS listing was also highlighted.

Section 2: Health technology funding and assessment pathways

Overall, there was broad “in principle” support for the options outlined in 2.1 and 2.2 of the Options Paper, and an acknowledgment from stakeholders of the opportunities that streamlining pathways and consolidating committees presented in achieving efficiencies in the HTA timelines and processes. Key concerns raised centred on the resource load this would place on the unified process, and the challenges of one pathway having the requisite breadth of knowledge to effectively assess the diversity of both current and emerging technologies (especially gene therapies, which are noted as a key omission from the reform options presented).

There was strong rejection across the pharmaceutical industry of a trade-off between price and an abridged/shortened HTA assessment process for cost-minimisation submissions. The proposed criteria of certain submissions needing to be lodged within six (6) months of receiving first regulatory approval from a comparable overseas regulator was also viewed as too restrictive.

In terms of the alternative options put forward for early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN, consumer groups commonly said that earlier engagement and resolution of issues was seen as offering greatest scope to reduce HTA delays. As such they tended to favour those encompassing earlier engagement (Options 1-3 in the Options Paper) – albeit with a view that price issues should only be considered as secondary to safety, efficacy and equity considerations.

Pharmaceutical and other stakeholders generally favoured Option 4 in the Options Paper given this provides greater certainty post evaluation – however several stakeholders queried how this option was vastly different to the current HTA process. A key issue of concern across the Options was the proposal to limit the number of resubmissions, which was identified as potentially resulting in fewer products being brought to market.

The concept of disease-specific models was supported by consumer stakeholders (pending appropriate input into their development) but were viewed as less useful among other stakeholder groups, some of whom pointed at limited success of such models in other jurisdictions.

Section 3: Methods for HTA for Australian government subsidy (technical methods)

There is support for the suggestions outlined in Option 3.1 in the Options Paper, with less discussion broadly across the submissions and minor points of difference identified between stakeholder groups. There is, however, a great deal of comment about the proposals in both 3.2 and 3.3 in the Options Paper. Both clinical evaluation and economic evaluation are talked about in depth throughout many of the submissions, and the points they raise are outlined and summarised below. Of note, there is also discussion in several submissions about the broader social value and environmental impacts of health technologies recommended for greater inclusion and consideration in HTA evaluation.

Those stakeholders that specifically mentioned the options under 3.1in the Options Paper, were all very supportive, particularly of the options to increase early stakeholder participation and to increase transparency, as they believed early engagement was critical to ensuring all of those who have the potential to benefit from a new therapy were included in the process.

Overall, there was support for updates to clinical evaluation methods, especially the consideration of and greater acceptance of consumer evidence, non-traditional data, Real-World Data (RWD) and Real-World Evidence (RWE), which many stakeholder groups believed would assist in the evaluation of new therapies. It was widely seen that these updates to clinical evaluation will improve the current methods - which were frequently viewed as being too narrow. Many also highlighted that flexibility and an increased level of comfort with residual uncertainty would be required to maintain a system that could keep pace with technology. Further guidance on how these specific reforms would be implemented and assessed was requested by a number of these groups.

There was quite a robust discussion in the submissions about economic evaluation and the proposals outlined in Option 3.3 in the Options Paper. There was broad discussion that the full economic value may not be reflected in the price that Government is willing to pay and/or that the negotiation of the price should be undertaken separately to the HTA assessment of cost-effectiveness. There were also discussions amongst stakeholders of a desire for broader economic evaluation encompassing additional factors such as environmental impact, ethical, wellbeing and societal benefit elements.

Section 4: Health Technology funding and purchasing mechanisms and decisions

Many patient groups were supportive of measures that could potentially make access to health technologies more affordable for their patients and the broader community. It was also highlighted that there needs to be a submission pathway open to non-commercial sponsors where there is no commercial imperative for a company, but there is critical clinical need amongst small population groups.

However, both of the reform options for cost minimisation submissions put forward at 4.1in the Options Paper were rejected unilaterally by pharmaceutical industry stakeholders on concerns such measures will see fewer products brought to market and ultimately limit patient choice. It was also commonly noted that a narrow focus on clinical efficacy and toxicity fails to recognise benefits such as improved quality of life and/or burden of treatment for patients that should be appropriately considered in the determination of value. It was argued that a matching price would be a more workable solution.

Another issue of contention centred on the reform option relating to post-market assessment. While acknowledging a need to continue to review performance of a funded technology in market, many suggested the current arrangements to facilitate this are sufficient. There were some concerns identified on the resource impost this could place on the HTA in terms of taking scarce resources from the assessment of new or emerging health technologies.

The concept of bridging funding held strong intuitive appeal for most. Pharmaceutical / Medical Technology Companies noted that more transparent and equitable risk sharing models would need to be developed to ensure the most innovative/uncertain technologies could leverage such a pathway.

The other reform options in this section were broadly supported across stakeholders, albeit with a common view that further consultation and co-design would be required if taken forward.

Section 5: Futureproofing our systems and processes

There is broad encouragement across the submissions for the options presented in 5.1 through to 5.5 in the Options Paper. Most stakeholder groups welcomed a more proactive approach to identifying therapies that address unmet needs, horizon scanning and strong support is also evident for increased environmental consideration. There were some concerns highlighted by Pharmaceutical / Medical Technology Companies about the options in 5.6 in the Options Paper, and they believed options such as international buying blocks would certainly not address the issues identified.

Stakeholder groups were generally very supportive of a proactive approach to addressing unmet clinical needs, developing a priority list, early engagement on the PICO and the options outlined for horizon scanning - many seeing this as one of the highest priorities. There were a number of comments about the need for widespread engagement and transparency on the development of a priority list for HUCN and in horizon scanning activities. The need for clarity was raised in relation to how the priority list would be selected and what diseases or conditions would qualify, particularly as there would be varying and competing priorities across consumer and patient groups. There was also a concern raised regarding the heavy reliance on sponsor-led submissions.

Overall, the potential greater inclusion of environmental impacts being considered in the HTA process was welcomed. A number of patient representative groups, peak bodies, clinicians and researchers highlighted the impact that the healthcare system has on climate change and the environment. It is also mentioned that climate change and increased pollution have a significant impact on the health and wellbeing of patients and consumers (with asthma sufferers put forward as a key example).

In futureproofing this system, many submissions focus on the need to consider environmental impacts through all stages of HTA processes. There was discussion about environmental impacts being reported throughout assessments and particularly as part of the cost- effectiveness considerations.

There was strong support amongst stakeholders for the suggestions in 5.4 in the Options Paper, particularly around transparency and improved forward planning of consultation and review. Many submissions mentioned continuous review and improvement as pivotal to the long-term success of the HTA, and to constantly be able to meet the needs of a rapidly evolving and technology driven system. As technologies and treatments change and are subject to innovation, the pharmaceutical and research stakeholders emphasised the importance of the system having adequate flexibility to accommodate assessment of these new technologies and explicit KPIs to track the success of any new reforms.

Throughout the written submissions, across a number of responses to the options, the capacity, capability and resourcing of the HTA system was mentioned. There were concerns raised in regard to the capacity of the HTA committees if streamlining were to be agreed upon and implemented, and there have been concerns raised about resourcing and capacity for horizon scanning to be introduced effectively and systematically. This meant there was general support for a review and overhaul of resourcing of the HTA system.

In some instances, stakeholder groups agreed that there were benefits from international partnerships and work sharing, but there were particular topics where groups highlighted some concerns. The Pharmaceutical / Medical Technology Companies did not endorse or see the benefit of international purchasing or buying groups, and there was a call generally across stakeholder groups for much more detail and consultation on these options.

Written submissions process

This section of the report summarises feedback received from stakeholders via the written submissions process.

HTA Reform Options Paper feedback survey

Stakeholders were invited to comment across on all reform options put forward in the HTA Reform Options Paper via an online survey. Stakeholders chose which parts of the HTA Reform Options they wanted to comment on in the survey, which is reflected in the varying bases sizes across the summary tables presented in this section of the report.

For all sections of the survey, stakeholders were asked two key questions about each specific reform:

- Taking all Options within this section into account: Overall, to what extent could the options (if implemented) address the issues that relate to them?
    - Completely address the issue(s)
    - Mostly address the issue(s)
    - Address some but not most of the issue(s)
    - Address little or none of the issue(s)
    - Don't know

- If implemented, overall would this option have a positive or negative impact on you (/your organisation)?
    - Very positive
    - Positive
    - Neutral
    - Negative
    - Very Negative
    - Don't know

Stakeholders were also asked to expand on their responses in open-ended questions. There was also an option to upload a separate written document as part of their response.

This section summarises these survey responses, including both the open text comments included alongside a response and any additional commentary provided in the separate written submission. Analysis has been undertaken by stakeholder group, with quotes included to highlight key themes and issues put forward in responses.

It is important to note that a Collaborative Consumer Group Response submission (representing the consolidated views of some fifty-one (51) consumer organisations) was provided separately to the survey, as was feedback from a range of other stakeholders who chose not to use the online survey. To this end, our base size in the tables do not capture these views – but certainly their feedback has been expressly noted across our analysis and commentary where relevant.

Section 1: Transparency, communication and stakeholder engagement

Stakeholders were invited to provide written comment on the reform options presented to improve transparency, communication and stakeholder involvement in HTA as per the table below (reproduced for the HTA Review's Options Paper).

Subject	Key option/s
1. Transparency, communication, and stakeholder involvement in HTA	
1.1. Transparency and communication of HTA pathways, processes and decisions	
Publish plain language summaries	Summaries of Pharmaceutical Benefits Advisory Committee (PBAC) submissions to be provided at the same time as the PBAC agenda is released to allow consumers (including patient communities and clinicians) to be better equipped to provide input to the HTA process and understand the expected benefit of the therapy and the proposed population without ambiguity (Note: this options does not seek to limit any outcome of the co-design of an Enhanced Consumer Engagement Process currently underway) Have clearer and more transparent description of the committee deliberations, including clear reasoning for recommendations / decisions made and what elements were included that is disseminated to broader stakeholder groups. Provide plain language explanation of the HTA pathways and PBAC guidelines that allow both experts and non-experts to be able to navigate the system more easily (with the level of information and language suited for the relevant audience levels).
Improvements to the HTA webpage including development of a dashboard	Have a visual dashboard including information to communicate the status of health technologies moving through the HTA system and HTA system performance statistics. Including information about timing of sponsor applications to overseas regulators, Therapeutic Goods Administration (TGA) and parallel pathway applications, PBAC submission and activities supporting PBS listing. This should be available at the aggregate and individual drug level and be informed by horizon scanning where possible. Make HTA websites easier to navigate accounting for different levels of knowledge.

1.2. Consumer, clinician and other stakeholder engagement and consideration in HTA

Develop an engagement framework	Development of an engagement framework which: establishes the inclusion of consumers, clinicians and other relevant stakeholders (such as ACCHO representatives) earlier and more consistently throughout the HTA processes including: horizon scanning, pipeline analysis, early assessment, Population, Intervention, Comparator, and Outcome (PICO) scoping workshops or pre-submission meetings to ensure that the PICO and HTA is addressing and including issues outcomes and populations relevant to consumers (for selected therapies), evaluation, appraisal committee, post market reviews, and disinvestment. describes how and why engagement with all stakeholders (with a particular focus on consumers) is used across all HTA processes and how engagement is used to co-design new processes and tools arising from the HTA review. integrates key outcomes of the *New Frontier* Inquiry Report, *Conversations for Change* consultation and report, the Consumer co-design project, and the HTA Review literature analysis and consultations. This would include the following: promoting consumer input into clinical trials and reduce duplication by asking sponsors to report any patient input or use of patient experience data in the research and development of the product public and consumer participant summary materials evolving from earliest engagement to final outcomes (including information about applications to support more targeted engagement) creating a patient/clinician HTA subcommittee to provide information to the HTA committee provide information, support, education and training to support more meaningful input reporting to groups about how their input has been used (such as through a values framework and briefings) inviting consumer inputs into how the technology is/will be used in the community (post-market reviews) adequate resourcing of proactive engagement: Address inequity of engagement by identifying consumer subgroups that do not engage with online portal and work with them to co-design appropriate engagement approaches clear and transparent guidance about how input should be prepared and is used by committees adoption of a consumer navigator for selected topics consumer participation in HTA committee meetings process for continuous improvement and review approaches for managing confidentiality and conflicts of interest
Strengthen consumer evidence	In addition to a consumer engagement framework, strengthen consumer evidence collection and utilisation by: adding additional guidance to the PBAC guidelines on the preparation and use of Real-World Evidence (RWE), consumer evidence (qualitative, Patient Reported Outcome Measures (PROMs, preferences, Patient Reported Experience Measures (PREMs) and equity in health (note this is detailed in clinical evaluation recommendations)

generating a curated list of methodologies that are preferred by decision-makers, including an explanation for consumers (note this is detailed in clinical evaluation recommendations)
working with a multi-stakeholder advisory group (including consumers) reporting to government, to co-design and oversee the development and implementation of enabling systems, pathways, evaluation, and research to optimise access and use of Real-World Data (RWD) in HTA. (including involving consumers to determine questions that can be addressed by RWD/RWE and involving consumers in the generation of data and co-design of communication materials)
establishing mechanism or methods to collate patient perspectives formally and routinely
including a feedback loop for consumer inputs to show how and where consumers have been consulted and how HTA committees considered this input
updating technical/committee guidelines to include methodological guidance (beyond the use of quantitative data) for committees and subcommittees to ensure there is a clear account of how consumer input is integrated and provide greater transparency on how committees consider consumer inputs.
Promote consumer input into clinical trials and reduce duplication by asking sponsors to report any patient input or use of patient experience data in the research and development of the product
Establish a dedicated consumer evidence base and condition/disease repository to develop specific measurement tools, collect relevant data for future HTA activities, and track patient outcomes and expectations over time
Include consumers in the HTA committee meetings: pilot real-time interaction to gain additional inputs required for deliberations and decision-making either before the committee meeting or during a more open part of the committee meeting (i.e. prior to committee deliberations).

1.3. First Nations people involvement and consideration in HTA

First Nations peoples partnership in decision-making	Establish a First Nations Advisory Committee to contribute to decision-making across the continuum of the below processes: Development of a priority list of population indications with high unmet clinical need (HUCN): In line with the priority reforms under the National Closing the Gap Agreement 2020 between all Governments and the Coalition of Peaks, a sub-set of the priority list (Refer to PAG – link) will be developed in partnership with Aboriginal and Torres Strait Islander community-controlled health services (ACCHSs) for the priority areas of HUCN for First Nations peoples. Horizon Scanning: An active horizon scanning process be developed to identify therapies with promising High Added Therapeutic Value (HATV) for indications on the priority list (this could include new therapies or new patient indications for the 'repurposing' of existing therapies) Proactive submission request for therapies that are on the priority list (see Proactively addressing areas of unmet clinical need and gaps in the PBS) Include a First Nations representative on the PBAC that can speak to specific benefits for and issues relating to First Nations peoples health Sponsor submissions to require consideration/assessment of the impact on health outcomes for First Nations peoples to enable meaningful informed decision-making.
Dedicated resource for HTA submissions and education	Have a dedicated resource for to assist organisations representing First Nations peoples health outcomes making HTA submissions including education and support for the submission development

1.4. State and territory government collaboration in HTA

Development of central standardised data sharing system for utilisation and outcome data	Increase collaboration through centralised data sharing and data standardisation (with funding for associated infrastructure) for utilisation and outcome data associated with use of health technologies to support nationally cohesive HTA.
Increase opportunities for consultation and work sharing	Promote more opportunities for input, consultation and work sharing by State and territory governments across the health technology lifecycle to support efficient and effective implementation and use of health technologies including providing State and Territory health departments opportunities for consultation and collaboration on HTA decisions that will have a significant financial or operational impact on them. (see also Capacity and capability in the HTA systems)

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)	Prioritise and expedite the development and implementation of a nationally cohesive approach to HTA as outlined in Schedule C of the 2020-25 National Health Reform Agreement (NHRA) Addendum. As detailed in the NHRA Addendum, this should include the development of a national HTA framework including processes for HTA to inform advice on implementation, investment and disinvestment opportunities at Commonwealth and State levels Establish timeframes for the implementation of high cost, HST funded through the NHRA with positive HTA recommendations to enable timeliness and equitable adoption of new technologies across Australia (modelled on the Key Performance Indicator for Government decisions with respect to the timeframes for listing medicines on the PBS) For example: within 2 months of in principle pricing agreement, an implementation plan at a national level to be published in collaboration with State and territory governments with the purpose to enable treatments to commence as early as 6 months. This should include transparency for the community with published information on the progress by all parties (Commonwealth, sponsor, and State and territory governments) Horizon scanning to facilitate timely planning and preparation for adoption by jurisdictions ahead of TGA application being lodged by the sponsor (see horizon scanning below) Establish (or participate in existing international collaboration) for Horizon Scanning, with input from a broad range of stakeholders including patient organisations, industry and State and Territory governments, particularly focused on high cost HST's funded through the NHRA, to ensure jurisdictions can begin early implementation planning of HST's Collaborate with the State and territory governments to ensure results of horizon scanning are being actioned into implementation plans. For potentially disruptive technologies, consideration of implementation requirements and initial implementation planning should occur simultaneously to the HTA with stakeholders encouraged to identify requirements for implementation within their HTA submissions (including sponsors, consumers, clinicians and State and territory governments): Establish a process to facilitate a collaborative mechanism for stakeholders to work together on implementation planning of a health technology early, including sponsors, State and territory governments, health practitioners and respective colleges to identify potential workforce and system capacity/capability issues and mitigation options (e.g. via education and training), to proactively support provisioning of new health technologies. See Proactively addressing areas of unmet clinical need and gaps in funded access Parties to the NHRA to develop a mechanism to reduce administrative burden and duplication for industry that occurs currently where sponsors are required to develop individual agreements with each jurisdiction and in many circumstances individual local health authorities.

Section 1 – Overall summary

There was strong support and encouragement across the majority of submissions for increased transparency, communication and consultation between stakeholders and the HTA, as well as State and Federal governments. Several written submissions also highlighted that a number of these recommendations could be implemented relatively quickly for immediate impact. It was also recommended by a large group of consumer representatives that the recognition of the value of the consumer voice be formalised through legislation, to ensure that it is safeguarded as part of the HTA. This group also highlighted that if the options in 1.1 to 1.3 in the Options Paper were progressed they would provide a fundamental platform for engagement to embed the consumer as a valued and equal contributor to the HTA process.

Across the stakeholder groups there was comment on the value that plain language summaries offered in assisting those with lower health literacy to understand the HTA process and the decisions ultimately made. Stakeholders also welcomed improvements and upgrades to the website and stated the introduction of a dashboard to the website would be a valuable new inclusion, especially in terms of increasing accountability on the progress of individual submissions.

There was general consensus amongst stakeholder groups that the options in 1.2 in the Options Paper were a step in the right direction for the HTA process and that increased involvement of stakeholders such as clinicians and consumers would lead to better outcomes for patients.

The vast majority of stakeholder groups see the potential that increased involvement and consideration could have to improve health outcomes for First Nations people. The criticality of supporting all initiatives that contribute to closing the gap and reducing the health inequities for First Nations people were emphasised and noted by all stakeholder groups. The need for KPIs to measure progress on these initiatives was also raised.

There was also a belief that increased and formal collaboration with State and Territory governments outlined in 1.4 in the Options Paper would assist with managing funding pathways and allow for better information sharing and the potential for a centralised data sharing system was supported broadly. The variations across States and Territories, as well as the need to negotiate individually with these jurisdictions, was raised as a concern that is not directly solved though these options. The need for a federally funded system for those therapies out of scope for PBS listing was also highlighted.

1.1. Transparency and communication of HTA pathways, processes and decisions

Table 1. 1.1. Transparency, communication and stakeholder involvement in HTA: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	73%	27%	0%	0%	30
Pharmaceutical / Medical technology company	0%	68%	26%	5%	0%	19
University or research sector	0%	50%	33%	17%	0%	6
Industry association / Peak body	0%	60%	40%	0%	0%	10
Clinician (or representative organisation)	0%	80%	20%	0%	0%	5
Consulting	0%	33%	67%	0%	0%	3
State / Territory government	-	-	-	-	-	0
Other	0%	75%	25%	0%	0%	4

Patients, Consumers and Representative Groups

The majority of patient representative groups were supportive of these proposed reforms given their scope to ensure the consumer/patient perspective was adequately considered in the HTA decision making process. Amongst the many patient and consumer groups who supported the increase in transparency, communication and consultation, there were a few who thought that these options could have gone further to address the issues. They believed that there is still work to be done to ensure all consumer and patient voices are actively involved throughout the entire HTA system. Some also believed that there was a strong need for co-design during the implementation of these options and highlighted the lack of funding for patient groups' participation in this work.

One of the patient representative groups commented that they *"strongly support the principle of unbiased plain language summaries and webpage improvements as a high priority. We believe this will go a long way to improving stakeholder engagement. We note that there needs to be a commitment to resourcing to achieve this. Consumers should be consulted on the development of the dashboard, HTA website improvements, and plain language summaries.* (Lung Foundation Australia)

"Our organisation invests hundreds of hours per year providing expert clinical information and facilitating consumer engagement (for nil compensation). We do not have commercial incentive, nor government support. We rely on the generosity of our donors and volunteer hours to make these contributions. This is not a sustainable model." (Myeloma Australia and the Medical and Scientific Advisory Group (MSAG))

One consumer representative group believed that *"the proposed options address only a subset of the identified issues, with a strong focus on the outward sharing of information. The document refers to stakeholders as a homogenous group however different stakeholders have different requirements. We represent consumers and assert that the proposed options in this section do not achieve the level of reform that is required. We need to consider authentic consumer engagement in the operation of the HTA process, not just the way in which HTA process information is shared. There is a power imbalance in the operation of the HTA process with the consumer voice largely missing. Stakeholder involvement needs significant reform, this must go further than how information is shared and putting a consumer representative or two on committees."* (PRIMCAT Consumer Panel)

It was noted by some that not all patient groups are represented and resourced equally– so this needs to be reflected in how patient groups are engaged through the process from a health equity perspective. This group *"welcomes the proposed reforms to improve transparency and communication of HTA pathways.* But they also noted "*that the aforementioned proposed reforms must ensure engagement of all relevant or affected stakeholders and mitigate any potential for uneven influence or an overreliance on the views of some individuals, organisations or groups."* This group "*considers that stakeholder engagement mechanisms must be designed to ensure that all relevant organisations, irrespective of size, have the capacity and opportunity to be involved in and participate in consultations.* (Painaustralia)

Another group suggested a further amendment to increase participation.

"Like the suggestions however it would be great if there could be a more proactive approach for consumers - such as push notifications to registered patient support groups or organisations like the GSNV who could then notify the relevant patient communities. Currently this is a time-consuming and not always comprehensive task to inform the community stakeholders. Responses could then be supported if required." (Genetic Support Network of Victoria)

Pharmaceutical / Medical Technology Companies

A number of pharmaceutical companies noted that while many of the methodological approaches to the conduct in HTA in Australia are reasonably consistent with other jurisdictions, Australia's HTA processes consistently deliver substantially lower assessments of value for innovative treatments versus comparable countries, despite starting from the same evidence base. While some noted that while many of the options proposed are likely to improve the experience of stakeholders engaging with the process – and are therefore supported – they may have limited impact on time to access without addressing this broader issue.

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

One of the consulting groups raised a broader concern here about whether the system was fit for purpose, and held concerns not just about engagement, but also the level of transparency and final impact on HTA decision making.

"Some of the problems, delays and stakeholder complaints about the current system stem from a lack of coordination, lack of transparency in evaluation and decision-making responsibility. Be they patients, industry, academics or even public sector officials, the lack of visibility and accountability about who is responsible for decision making in government is one of the growing

problems in the current system. Ultimately, the accountability and transparency of government decisions to decide whether to fund or not to fund a medical technology could be better. The current system might have been appropriate 30 years ago when HTA evaluation was first introduced in Australia, but given the growth in scope, scale, professionalism, and influence of HTA in the health system today, the systems supporting HTA need to change." (Shawview Consulting)

A peak body highlighted their support but made suggestions on the reforms needing to go further. *"We generally support initiatives to improve public engagement in the HTA process and would support further application of these improvements across HTA for the full IVD sector."* (Pathology Technology Australia)

There were few unintended consequences of these reforms noted across the written submissions, albeit many stakeholders expressed a degree of concern of how resource intensive the production of the plain language summaries and the dashboard website would be. It was noted that the provision of these additional resources – without a commensurate increase in funding/resourcing - could divert assessment resources away from the HTA process and potentially impact on the timely assessment of HTA submissions.

Table 2. Publish plain language summaries – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	4%	0%	4%	32%	61%	0%	28
Pharmaceutical / Medical technology company	0%	0%	16%	68%	11%	5%	19
University or research sector	0%	0%	50%	33%	17%	0%	6
Industry association / Peak body	0%	0%	20%	50%	30%	0%	10
Clinician (or representative organisation)	0%	0%	0%	75%	25%	0%	4
Consulting	0%	0%	0%	67%	33%	0%	3
State / Territory government	-	-	-	-	-	-	0
Other	0%	0%	40%	20%	40%	0%	5

There was a very positive and supportive response from all stakeholder groups to the option to publish plain language summaries. Most also believed that this option could be implemented straightaway to provide immediate impact and improvement, particularly for patients and consumers.

Patients, Consumers and Representative Groups

A number of consumer and patient representative groups provided comment on how the reforms could go further to improve the involvement and participation of critical stakeholders in the HTA process.

One patient representative group identified that *"plain language is essential to consumer understanding of health matters but it is also essential that consumers health literacy is increased and that they have access to detailed information that will assist with this. Clear, concise language accurately reflecting reasoning, decision and actions that organisations like [ours] can utilise to better inform consumers will naturally support other activities to increase consumer health literacy.* (Anonymous submission)

Further to this another group emphasised the need to understand how these would be developed - and by who.

"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." (Rare Voices Australia)

A number of these groups also commented that this option will vastly improve transparency for consumers and patients.

"Publishing plain language summaries enables transparency and equity of consumer access in the context of diversity in health literacy. Current PBAC Agenda listings do not provide sufficient information for consumers." (Australian Patient Advocacy Alliance)

"BCNA would welcome changes to how HTA processes and outcomes are communicated to improve accessibility and enhance the ways in which a diverse range of consumers can input into HTA and be informed." (Breast Cancer Network Australia)

In addition to plain language summaries, come consumer groups also called for more timely and meaningful engagement and guidance such that consumers can better understand what inputs are required of them, in what format, and within what timeframes.

"We support this option to improve consumer access to understand what the application is seeking and enable them to provide relevant feedback. Current one line PBAC Agenda listings do not provide sufficient information for consumers to understand what is being sought through the application e.g. is it a novel agent with a different therapeutic action, is it a different preparation with administration differences. (Crohn's and Colitis Australia)

Pharmaceutical / Medical Technology Companies

Most pharmaceutical company stakeholders were supportive of this proposed reform, albeit with comments on resourcing and confidentiality needing to be considered.

One pharmaceutical company commented that they supported the implementation of plain language summaries for PBAC submissions. This company *"participated in the Summary of Information pilot and actively supports efforts to ensure patient organisations, and in turn patients, have information that facilitates their ability to provide input into HTA decision making and further, to support robust decision making"*. They also believed *"that criteria are needed to define which submissions are appropriate and would benefit from formal summaries and does not advocate that all submissions require a summary."* (Bristol Myers Squibb)

While several companies indicated support for the publishing of plain language summaries of the patient/population, intervention, comparison, outcomes (PICO) at the time that the PBAC agenda is released, there was a strong need for all private pricing information and company information to remain confidential.

"All elements relating to net pricing and risk share arrangements must remain commercial-in-confidence." (Alexion)

"We support initiatives that improve patient and clinician engagement in the process while ensuring time to access is not increased through the implementation of this initiative. A key caveat to this is that information that is considered confidential under the status quo remains confidential in any new transparency arrangement. A particular concern is that special pricing arrangements continue to be a policy option in any future arrangement and that confidentiality around special pricing arrangements is preserved in any future model. Further, the ongoing issues with redactions in PSDs must be addressed." (Eli Lilly Australia)

"Published plain language summaries are very worthwhile and have been successfully implemented in other markets around the world. There does need to be a consistent template applied by all sponsors that remains accessible for consumers/stakeholders and is relevant and short in page numbers. Knowing the content of the plain language summaries would be helpful in order to make an informed decision about their usefulness. Confidential company information should not be included." (Antengene)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Amongst the comments from these groups there was praise for this option and some highlighted that it had the potential to increase trust and be a very strong start for the processes of the HTA in becoming truly 'patient-centric'. Similar to the stakeholder groups already highlighted above, there was also some discussion about how these summaries would be developed.

"*Publishing PBAC guidelines also enhances consistency and clarity in decision-making. Clear guidelines help stakeholders navigate the HTA process, ensuring uniformity in submissions and evaluations. This reduces ambiguity and improves the efficiency of the HTA process, leading to timely access to innovative therapies for patients. Furthermore, the dissemination of plain language summaries and guidelines supports education and capacity building among healthcare professionals. By providing clear explanations of HTA processes and criteria, PBAC equips healthcare professionals with the knowledge and tools needed to engage effectively in the decision-making process, thereby promoting evidence-based practice."* (Society of Pharmacists of Australia)

"Publishing plain language summaries is critical in any truly 'patient-centric' process. It must never be assumed that consumers are familiar with complex scientific language or with government decision making processes. Patients deserve not only a plain-language summary - a modern HTA system must also demonstrate to patients how their submissions have been considered and they should be reassured that their experience has been appropriately taken into account. This can happen via the provision of full meeting minutes or by enabling the parts of a meeting where patient submissions are being considered to be public." (Specialised Therapeutics)

"I would welcome plain language summaries in order to make our entire process (from sponsors to evaluators to decision makers) more accountable. I would like these plain language summaries to follow a set structure (obviously in consultation with the relevant stakeholders) so that the plain language is useful, repeatable and reliable. That is, I wouldn't want it to be a summary of the HTA process with the jargon taken out. Rather, themes such as "What was claimed", "What was proven", "What was valued" are consistent across most of our submissions and can be answered with relatively plain speaking without the need to describe the heterogeneity in some obscure indirect treatment comparison. I wouldn't want the plain language summaries to in any way substitute for the technical information that is often important for understanding PBAC decision making when preparing submissions." (THEMA Consulting)

Table 3. Improvements to the HTA webpage including development of a dashboard – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	11%	29%	61%	0%	28
Pharmaceutical / Medical technology company	0%	0%	32%	53%	11%	5%	19
University or research sector	0%	0%	33%	50%	17%	0%	6
Industry association / Peak body	0%	0%	10%	60%	30%	0%	10
Clinician (or representative organisation)	0%	0%	25%	25%	50%	0%	4
Consulting	0%	33%	0%	33%	33%	0%	3
State / Territory government	-	-	-	-	-	-	0
Other	0%	0%	20%	60%	20%	0%	5

Patients, Consumers and Representative Groups

Again, there was broad encouragement and support for this option. A number of patient and consumer representatives noted the importance of improving the searchability of information on the site, with a view that current processes were cumbersome and time intensive (e.g. needing to physically open and search for medicines or applications in individual submissions, as opposed to being able to conduct a search across all submissions simultaneously). These stakeholders also hoped that further engagement in a co-design process would help manage some of these issues and ensures any new functionality meets the needs of all users, including multicultural communities for whom English may not be a first language.

One patient representative group highlighted *"improvements to the HTA website and proposed dashboard would be a welcome improvement to information dissemination and understanding of the community, as well as improve transparency around processes and options for input from a stakeholder perspective"* They also noted that *"in order to provide and develop a website and dashboard hub that meets the needs of consumer and stakeholder organisations, it is vital that*

this development is undertaken with thorough input and engagement from those with lived experience." (Ovarian Cancer Australia)

"The format and content for plain language summaries and changes to the website must be co-designed with all stakeholders to optimise the changes." (Genetic Support Network of Victoria)

"The HTA webpage needs to cater to Australia's multicultural community by providing content in multiple language, as well as providing options for various accessibility needs (such as vision impairment)." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

Again, there was very broad support from these companies for website upgrades and improvements. There was not a great deal of discussion specifically on this option but one company highlighted that they are supportive and *"also believe that establishment of a visual dashboard which tracks the status of health technologies in the HTA evaluation process, along with HTA system performance statistics would have a positive impact. These statistics need to include HTA evaluation performance KPIs that are co-designed with key HTA system stakeholders. Any changes in transparency and communications should retain current facets of the HTA process such as special pricing arrangements and confidential pricing, that allow Australians to access innovative medicines.* (AstraZeneca)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was also general support from these groups. One group commented on the value of the dashboard, but another highlighted that if the HTA system achieved its reforms, the dashboard may not be necessary (or at least represent a more simplified and streamlined process).

"The development of a visual, data-driven dashboard for the HTA webpage offers significant benefits, including increased transparency, evidence-based decision-making, accountability and stakeholder engagement. These improvements contribute to a more efficient, equitable and responsive HTA system, ultimately improving patient access to high-quality healthcare technologies in Australia. To be honest, and is probably a bit idealistic, but I think I would prefer a HTA process that was simple enough not to require the development of a dashboard. Maybe the dashboard could come after some of the proposed refinements to the process are implemented." (Shawview Consulting)

1.2. Consumer, clinician, and other stakeholder engagement and consideration in HTA

Table 4. 1.2. Consumer, clinician and other stakeholder engagement and consideration in HTA: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	3%	72%	14%	0%	10%	29
Pharmaceutical / Medical technology company	0%	65%	25%	10%	0%	20
University or research sector	0%	60%	0%	40%	0%	5
Industry association / Peak body	0%	80%	20%	0%	0%	10
Clinician (or representative organisation)	0%	60%	40%	0%	0%	5
Consulting	0%	25%	25%	0%	50%	4
State / Territory government	0%	0%	100%	0%	0%	1
Other	20%	40%	20%	20%	0%	5

There was general consensus amongst stakeholder groups that the options in 1.2 in the Options Paper are a step in the right direction for the HTA process and that increased involvement of stakeholders such as clinicians and consumers would lead to better outcomes for patients.

Patients, Consumers and Representative Groups

Patients and consumer groups overwhelmingly support the options in 1.2 in the Options Paper. One patient representative group commented that they *"support the involvement of consumers, clinicians and other relevant stakeholders in the development of an engagement framework. Whilst we recognise the diversity of stakeholders relevant to the HTA process, the consumer must have true equity as a key stakeholder"*. (Australian Patient Advocacy Alliance)

Further comments of support and encouragement for the adoption of this option were *"Consumer engagement throughout the HTA process is essential for delivering the best outcomes for patients. MSCAN welcomes the all the options outlined in the Options Paper to engage stakeholders earlier and more consistently throughout the HTA processes".* (Melanoma and Skin Cancer Advocacy Network (MSCAN)

"The development of the engagement framework we endorse. However, it must have consumer input mandated as a KPI. There would be significant benefits to this suggestion and support improved outcomes. This is especially important in the rare diseases space where there are smaller population groups. Their input is as important as the larger groups. More detail is required on these suggestions and how they would work but conceptually this is helpful progress." (Cystic Fibrosis Australia)

Pharmaceutical / Medical Technology Companies

The majority of Pharmaceutical / Medical Technology Companies supported the options in 1.2 in the Options Paper. Most believed they would address the issues and improve consumer engagement in HTA processes. There was emphasis on the importance of co-design for this to be successfully implemented.

"Roche supports the development of a consumer engagement framework and the proposed mechanisms for strengthening consumer evidence collection and utilisation. The increased input will improve the person-centredness of decision-making. Roche acknowledges the work of the HTA Consumer Consultative Committee (CCC), the Department's CEEU and the Co-design working group of the HTA CEEU and the Patient Voice Initiative that has been progressed to date. Overall, Roche supports the Options covering Chapters 1.2. Roche recognises a significant level of co-design and creation, inclusive of industry, is required to expand on the specifics of the options to ensure that the issues can be more completely addressed." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was broad support for these options amongst these stakeholder groups and they believe increased consumer engagement was critical. There was comment about the need for increased emphasis on clinician, primary care and frontline workers to ensure the best outcomes for patients, as these workers are the trusted advisers. One group also called for the need to explicitly call out children as a stakeholder group worthy of specific and specialised consideration.

"Clinician engagement needs to be expanded, while it is very important to have consumer's input the paper lack measurable ways in which clinician input can be sought. This is paramount when assessing new technologies in rare conditions, or in large population where clinical advancements have been limited and data is also limited. It is also critical to get input in areas where performance of technology is being discussed when it is an early adoption or driver change in the therapy area. this can be done by establishing clinician advisory groups who then advise the committee on various aspect of the submissions/ technology and it's performance including ways generate RWD if none exist." (Consultant)

"These need to include the needs of the paediatric population, with facilities for their voice to be heard." (Monash Children's Hospital)

"Need third party consultation. primary care and front-line workers need to be more involved because they need to deliver the programs. They need to be able to trust the advice and sources and there needs to be transparency." (Immunisation Coalition)

Table 5. Develop an engagement framework – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	4%	30%	63%	4%	27
Pharmaceutical / Medical technology company	0%	0%	10%	80%	10%	0%	20
University or research sector	0%	0%	25%	75%	0%	0%	4
Industry association / Peak body	0%	0%	9%	73%	18%	0%	11
Clinician (or representative organisation)	0%	0%	20%	60%	20%	0%	5
Consulting	0%	0%	25%	50%	0%	25%	4
State / Territory government	0%	0%	0%	100%	0%	0%	1
Other	0%	0%	40%	40%	20%	0%	5

There was not a single stakeholder group who believed this option was negative. Some remained neutral, but the vast majority saw an engagement framework as a positive step. There was some comment across multiple groups about the need for clarity and detail about the scope of the framework and stakeholder participation, equitable representation across a vast majority of groups (patients, clinicians, front-line workers, sponsors, etc) and the significance of the framework being co-designed with all stakeholders.

Patients, Consumers and Representative Groups

There was widespread support for an engagement framework from these groups - they believed that further consumer input and engagement would strengthen health policy development and outcomes. One group highlighted that they would need more information on how the framework would be established and what its features would be before they could comment on whether or not it would be a positive option.

"MSCAN welcomes the Options outlined to develop an engagement framework. In particular, we acknowledge the importance of a framework that describes how and why engagement with all stakeholders is used across all HTA processes. We note the particular focus on consumers." (Melanoma and Skin Care Advocacy Network)

"It is difficult to comment on whether the development of an engagement framework will be positive or negative without more information on how the engagement framework will be established and what its features will be." (PRIMCAT)

Pharmaceutical / Medical Technology Companies

These companies overwhelmingly supported this option and believed it was critical to ensure that consumer voices were part of the decision-making process. One company did flag their concern that the inclusion of the voice of the sponsor is not specifically addressed here.

"CSL welcomes the intention to increase stakeholder engagement with the HTA process by developing a framework to include consumers, clinicians and other stakeholders more consistently throughout the HTA processes (1.2.1). However, we are concerned that the framework outlined in the proposal does not specifically address the inclusion of submission sponsors. It is also unclear how ongoing dialogue between the sponsor, submission evaluators (clinical and economic) and advisory committees (e.g. ATAGI) will be incorporated in the framework." (CSL Limited)

"We support the development of a consumer engagement framework to ensure that the voices of those directly impacted by the technology are part of the decision-making process. We hope this will lead to health technology assessments that reflect real-world needs and priorities, and equitable outcomes. Consumer input can also reveal practical considerations often missed by technical experts such as usability, accessibility and potential social impacts. In the final options paper, the pharmaceutical industry should be identified as a stakeholder who should have an active role in the co-design." (UCB Australia)

"Biogen supports the development of an engagement framework, as it brings consumers and patients closer to the process." (Biogen)

Peak Bodies, Clinician/Researchers, Consultants, Not-For-Profits (NFPs)

These groups also supported this option but believed that the framework could be strengthened for the inclusion of clinician and expert input.

"A model such as a community of practice can be very beneficial and can pool all the strengths and knowledge of each discipline together." (Australasian College for Emergency Medicine)

"Development of a formal engagement framework with organisations such as MOGA (slightly more than what is currently done) would be an option to improve expert input in key priority areas such as oncology drugs and tests." (Australian Centre for Accelerating Diabetes Innovations)

"Strengthen the framework for clinician input." (Shawview Consulting)

"Consultation with all the stakeholders from government to consumer. Note the importance of WHO, Government (all levels including local gov), pharmacy, mobile workplace vax, GPs, wholesalers – supply chain, nurses." (Immunisation Coalition)

State and Territory Government / Departments

"The proposed framework would be an important first step in improving stakeholder engagement with HTA. A particular issue for public health interventions such as population screening is engaging and understanding the views of a broad, informed public, and incorporating the views and experiences of people who (usually in a research/trial setting) have received false positive screening results or uncertain results." (Department of Health, Western Australia)

Table 6. Strengthen consumer evidence – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	7%	15%	74%	4%	27
Pharmaceutical / Medical technology company	0%	0%	16%	63%	16%	5%	19
University or research sector	0%	0%	25%	50%	25%	0%	4
Industry association / Peak body	0%	0%	9%	64%	27%	0%	11
Clinician (or representative organisation)	0%	0%	0%	60%	40%	0%	5
Consulting	0%	0%	50%	25%	25%	0%	4
State / Territory government	0%	0%	0%	100%	0%	0%	1
Other	0%	0%	60%	40%	0%	0%	5

Even though there was broad support from all stakeholder groups for strengthening consumer evidence there was some commentary from groups about:

- the extent to which the evidence will be taken into consideration during decision making processes,
- the types of evidence taken into consideration, and
- the transparency of the decision-making process.

There was also a comment about the criticality of early patient involvement, data privacy and funding for resourcing of these activities for organisations with limited capacity.

Patients, Consumers and Representative Groups

There was a very positive response from these stakeholder groups to the option to strengthen consumer evidence. Some of these groups questions the influence that this evidence would have on decision making and requested more information on this. One group also raised the issue that additional resourcing may be required by smaller patient representative groups to support this.

The Collaborative Consumer Group Response commented that as part of this option they supported the idea of creating a patient/clinician HTA subcommittee to provide information to the HTA committee, but only if it is not a replacement for the consumer members on HTA committees.

"With respect to strengthening that consumer evidence however, we would suggest a stronger mandate is needed to involve patients up front. This could be achieved, as has been done in other jurisdictions, by a requirement to explicitly outline the involvement of patients during the clinical trial phases, as well as their experiences of the product during clinical trials (beyond

clinical outcomes) when applying for registration and reimbursement of their products. Requiring such evidence would also increase certainty for the HTA review committees, should they be considering provisional funding arrangements at the time of registration, that a product indeed is desirable, safe, effective and addresses patient needs, from the patient and family perspectives." (AccessCR)

"Ovarian Cancer Australia was recently involved in two lengthy concurrent submissions assessed by PBAC and MSAC. There were some challenges to overcome before the committees felt comfortable to recommend subsidies of the test and medicine. Ovarian Cancer Australia offered to have lived experience representation at the meeting to support the decision-making process, an offer that was declined. We see this as a potential area where lived experience may have played a critical role and aided in the timing and comfort for these submissions. Ovarian Cancer Australia welcomes the proposed options to reform within this area." (Ovarian Cancer Australia)

"Resources need to be made available for consumer input, both training, and reimbursement for time. Not all patient organisations or individual consumers have the capacity to do this important work without funding being made available. This will ensure that input is equitable." (Childhood Dementia Initiative)

"A key concern is the opaque understanding of the value of stakeholders evidence, including evidence from consumers and their representative groups. In many cases, the questions in HTA require a scientific and/or medical assessment based on scientific and medical evidence, and potentially health economic evidence. To what extent can (and should) the views of other organisations contribute to this decision-making? Until this question is clarified and communicated, it is difficult to understand the value of our participation in these processes, and what tools should be used to make the pathways, processes and decisions more transparent. We would encourage greater stakeholder engagement (with health providers such as CDEs and endocrinologists as well as people living with diabetes and consumer groups) to ensure the feedback into the decision-making process is robust and comprehensive." (The Australian Diabetes Alliance)

Some patient advocate groups reiterated concerns that a more onerous engagement process may pose equity challenges between disease or therapy areas that have differing levels of resources and capacity to engage. It was argued that stakeholders needed to be supported to contribute equally and equitably.

Pharmaceutical / Medical Technology Companies

Although very supportive of this option, some Pharmaceutical / Medical Technology Companies requested clearer guidance on the types of evidence PBAC would consider appropriate, and requested that the pilot program for patient involvement pave the way for sponsor presence. A concern about data privacy was also raised by one company.

"(AbbVie would like to see the pilot program for patient involvement in PBAC meetings pave the way for Sponsor presence, to support greater transparency around decision making and the PBAC's assessment of consumer evidence and the value of broader qualitative evidence." (AbbVie)

"To support consumer advocacy groups, clear guidance is required on the types of evidence that the PBAC would consider appropriate." (Boehringer Ingelheim)

"Consumer consultation is essential to ensure the creation of a consumer evidence base meets consumer expectations especially with respect to data privacy. The same is true for the creation of a centralised data sharing system for utilisation and outcomes data for HSTs. Consideration of who can access this information is also important to ensure the new data created supports the goals of the review in achieving faster access." (Pfizer)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

A large number of these stakeholder groups articulated their support for this option, but there were some suggestions to broaden this approach to include clinicians and other experts. They highlighted some additional challenges that will be faced to build this evidence base, including resourcing constraints. One group also wanted to ensure that there was a mechanism for engaging the most appropriate representatives for the evidence base - representatives with an acute understanding of the issues facing that specific population.

A peak body for GPs advocated the importance of consulting with and considering the impact on health providers *"including input from GPs, as specialist generalists, into evidence-to-decision frameworks helps prevent an excessive focus on a single organ system and narrow outcomes. GPs provide a holistic perspective to care and this broader perspective enhances the comprehensiveness of decision-making processes".* Early engagement and advance notice were highlighted as crucial as *"this not only ensures transparency but also allows GPs to provide valuable input and make necessary prescribing changes, especially considering that some GP prescribing occurs at intervals of 6-12 months for patients whose condition is stable."* (RACGP)

"*We would like to reiterate that Australian HTA can be time-consuming and costly, especially for small organisations. For example, in the case of the IPAC Trial, the sponsor surmised that the cost of the HTA through MSAC may have exceeded the cost of the Commonwealth-funded IPAC Trial evaluation itself. We support HTA generally being more flexible, and expansion of the range of evidence (especially real-life studies) being assessed."* (NACCHO)

"While I commend the inclusion of consumers on the MSAC committee, currently it is not clear that the consumer is the most appropriate person to be providing comment. For example, if this is a technology aiming to improve health outcomes for either a specific population such as Indigenous people, or persons with a particular illness such as Hepatitis C, then the consumer talking to the MSAC committee should be a patient or community representative who actually is acutely aware of the issues for that specific population. While it is the norm for letters of support to be provided, it is not the same as a providing the opportunity for a person who will be directly impacted by the decision to be part of the MSAC committee discussion." (Anonymous submission)

1.3. First Nations people involvement and consideration in HTA

Table 7. 1.3. First Nations people involvement and consideration in HTA: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	64%	21%	0%	14%	14
Pharmaceutical / Medical technology company	7%	86%	7%	0%	0%	14
University or research sector	0%	25%	25%	25%	25%	4
Industry association / Peak body	0%	67%	22%	0%	11%	9
Clinician (or representative organisation)	0%	50%	0%	25%	25%	4
Consulting	-	-	-	-	-	0
State / Territory government	-	-	-	-	-	0
Other	0%	60%	40%	0%	0%	5

There was a great deal of encouragement and support in regard to these options. The vast majority of stakeholder groups see the potential that increased involvement and consideration could have to improve health outcomes for First Nations people. The criticality of supporting all initiatives that contribute to closing the gap and reducing the health inequities for First Nations people were emphasised and noted by all stakeholder groups. The need for KPIs to measure progress on these initiatives was also frequently raised.

Patients, Consumers and Representative Groups

These groups were overwhelmingly supportive of this option.

"The NAA supports all of the proposals in this section that are intended to lead to improved outcomes for First Nations people. Once again, KPIs will need to be developed to measure progress." (Neurological Alliance Australia)

"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." (Rare Voices Australia)

"All parties must be culturally aware and work and work to ensure that we are meeting and collaborating the needs of First Nation People. Without this consideration the involvement in HTA of First Nations People will not occur or be accepted." (NeuroEndocrine Cancer Australia)

"Ovarian Cancer Australia welcomes the opportunity within the options paper for a more equitable and formal approach to engagement with First Nations peoples. This may provide the

opportunity whereby an organisation may not have already engaged with consumers with First Nations backgrounds to provide input and have greater engage in these areas, ensuring more equitable outcomes for all. If we are to truly represent the interest of all Australians, and create a fair and equitable landscape, then reform within this area is critical for change." (Ovarian Cancer Australia)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies also showed a high level of support for this option. There was a strong push for First Nations people involvement and continuous engagement with NACCHO.

"Novartis recognises the importance of considering the impact of health technologies on Aboriginal and/or Torres Strait Islander peoples. If a health technology is identified as potentially benefitting these communities, seeking advice from representatives of Aboriginal and Torres Strait Islander community-controlled health services early in the process will ensure that that the voices and needs of our First Nations Peoples are included. It is important that this perspective is considered by the PBAC in its deliberations as frequently specific evidence within this population will not be available for any submission. In addition, the valuation of any impact from a health technology needs to consider the ability to narrow the health inequality gap that is so harmful to improving longer term outcomes in these communities." (Novartis Australia)

"We are highly supportive of initiatives to increase the involvement of First Nations people in HTA and the aim of improving access to Health Technologies for areas of unmet need for First Nations peoples. Consistent with the proposals for horizon scanning, a clear framework is essential to ensure that Sponsors are clear on the process and expectations for proactive submission requests for First Nations people." (Pfizer)

"BMSA supports all closing the gap initiatives and recognises the tremendous issues facing Indigenous Australians healthcare. BMSA would encourage the Committee to consider criteria for sponsor submissions requiring considerations and assessment of impact for First Nations people. Noting however, the ability to impact clinical trial protocols is limited for local affiliates of multi-national companies, and as such, applicable data may be limited." (Bristol Myers Squibb Australia)

"We strongly support equitable outcomes as a pillar of the Options paper with the inclusion of First Nations people involvement in HTA. We would have also liked a stronger call-out to other marginalised groups including those with rare or ultra rare conditions." (UCB Australia)

"Roche is supportive of improving First Nations people involvement and consideration in HTA, and establishing dedicated resources to support HTA education and submission development. Roche believes that First Nations people and their representatives are best placed to comment on these proposals, and Roche is willing to work in partnership with First Nations people and their representatives on the proposed options, when and where appropriate. Given the expertise the health technology industry can contribute to this option, consideration should be given to its involvement in supporting submission development, as well as potential arrangements for repurposing and proactive submission requests. Roche notes a potential future role for the proposed National Aboriginal Community Controlled Health Organisation (NACCHO) and

Medicines Australia (MA) Health Equity Collaboration in furthering the options to address the identified issues." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was also strong support from these stakeholder groups, who identified a number of the challenges currently facing First Nations people. NACCHO highlighted that under this Section of the Options Paper there may be an avenue to deal with custodianship of the current section of the PBS for Aboriginal and Torres Strait Islander people.

"We acknowledge that the current section of the PBS that outlines PBS medicines for Aboriginal and Torres Strait Islander people. We support such a list conceptually and acknowledge the impact it has had in improving access to medicines for First Nations people for a couple of decades, including areas where there is high unmet clinical need. However, the current list has no real custodianship. It exists through implicit mechanisms and vague incentivisation. While we realise that sponsors may receive additional support (i.e. those currently outlined in PBAC guidelines and procedures) in applying for a listing such items, priority medicines for our sector are not manifestly being listed and several critical medicines have recently been delisted. We provide several reasons for this in our previous submission, which may be considered as cases to adjudge the potential effectiveness of the Options in this current paper. For example, whether nicotine replacement therapy for Aboriginal and Torres Strait Islander people would be listed through these Options. We feel the most effective way to manage such a list is outlined broadly within section 1.3." (NACCHO)

"A major issue impacting on First Nations peoples is their inability to access reduced co- payments of PBS medicines upon discharge from hospital. The proposed options in this paper do not address this significant issue that leads to lack of treatment poorer health outcomes for Aboriginal and Torres Strait Islander people. Hospital pharmacists must be enabled to supply medicines to Indigenous Australians under Closing the Gap PBS Co-Payment Measure. (Society of Hospital Pharmacists of Australia)

Table 8. First Nations peoples partnership in decision making – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	14%	71%	14%	14
Pharmaceutical / Medical technology company	0%	0%	13%	47%	33%	7%	15
University or research sector	0%	0%	20%	40%	0%	40%	5
Industry association / Peak body	0%	0%	0%	44%	44%	11%	9
Clinician (or representative organisation)	0%	0%	0%	50%	25%	25%	4
Consulting	-	-	--	-	-	-	0

State / Territory government	-	-	-	-	-	-	0
Other	0%	0%	20%	40%	20%	20%	5

Stakeholder groups again overwhelmingly supported initiatives to improve health outcomes for First Nations people. Many believed that including them in the HTA decision making process could positively contribute to this. Across the groups they highlighted that exploring barriers to access and continuing to work closely with NACCHO were vital to closing the gap, a formalised framework approach was suggested by a few stakeholder groups.

Patients, Consumers and Representative Groups

There was a very high level of support for this option from these stakeholder groups. They emphasised the widening level of health disparities between Indigenous and non-Indigenous Australians and the urgent need for major reform to deal with this. One group also requested more detail on implementation and specifically how partnerships with community-controlled health organisations could be leveraged here.

"BCNA fully supports greater participation of First Nations peoples in HTA decision making. We would like to see further details regarding implementation, including how partners with community-controlled health organisations, could be leveraged to reach this goal." (Breast Cancer Network Australia)

"CHF welcomes and supports better involvement of First Nations peoples in HTA processes. News of the widening of the health disparities in Australia between Indigenous and non- Indigenous populations are alarming and reveal the great need for ambitious health reform - HTA included. CHF supports the creation of a specific sub-set of the priority list which will be dedicated to areas of high unmet clinical need specifically for First Nations Peoples." (Consumer Health Forum of Australia)

"Solutions to address First Nations peoples effected by rare cancer need to be identified as there is already inequitable access to routine standard of care therapeutics." (Australasian Leukemia and Lymphoma Group)

Pharmaceutical / Medical Technology Companies

Very strong support was seen from these companies for this option.

"We are highly supportive of initiatives to increase the involvement of First Nations people in HTA and the aim of improving access to Health Technologies for areas of unmet need for First Nations peoples. Consistent with the proposals for horizon scanning, a clear framework is essential to ensure that Sponsors are clear on the process and expectations for proactive submission requests for First Nations people." (Pfizer)

"In Roche's experience working with NACCHO, establishing a partnership with First Nations peoples in HTA (and other) decision making processes is a positive step towards supporting self-determination and the widely endorsed principle amongst First Nations peoples of "Aboriginal health in Aboriginal hands". (Roche Products)

"Biogen broadly supports the partnership of First Nations People in decision making." (Biogen)

Peak Bodies, Clinician/Researchers, Consultants, Not-For-Profits (NFPs)

There was broad and strong support for this option from these groups.

"A sustained process is needed - the current PBS list for Aboriginal people was established 20 years ago and now has no real oversight." (NACCHO)

"Having pre-established accessible information about specific needs for First Nations populations will make it easier for sponsors to tailor submissions." (Medical Technology Association of Australia)

"The establishment of a dedicated Advisory Committee and including a representative on the PBAC, ensures that First Nations perspectives and priorities are integrated into decision-making processes from the outset. This promotes cultural sensitivity, inclusivity, and responsiveness to the unique health needs and priorities of First Nations peoples." (Society of Hospital Pharmacists of Australia)

"Health equity and accessibility of healthcare are important considerations in HTA, to help ensure that decisions regarding the adoption of interventions do not increase health inequalities of First nations Australians. Current HTA method guidelines do not require the quantification of the health impacts on Aboriginal and Torres Strait Islander people. A standardised approach to systematically quantify health inequities for First Nations Australians would enable the comparison of the impact across different interventions and health conditions and could facilitate HTA adopting a more transparent and rigorous strategy to ensure that health inequalities between Indigenous and non-Indigenous Australians are not increased." (Deakin University)

This University also highlighted that an approach that could be used "distributional cost-effectiveness analysis (DCEA) is a method that can provide quantitative information about the overall equity impact of funding new health technologies and the trade-offs that may arise between equity and efficiency (health maximisation). DCEAs can quantify the distribution of expected health benefits of interventions by Indigenous and non-Indigenous status. This methodology enables an intervention to be classified as cost-effective or not cost- effective and reduce or increase health inequality." (Deakin University)

State and Territory Governments / Departments

State and territory stakeholders were also supportive of this proposed reform, albeit with calls to ensure transparency on how any First Nations input actually impacts on final HTA decision making.

Table 9. Dedicated First Nations resource for HTA submissions and education – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	21%	64%	14%	14
Pharmaceutical / Medical technology company	0%	0%	13%	53%	20%	13%	15
University or research sector	0%	0%	40%	40%	0%	20%	5
Industry association / Peak body	0%	0%	0%	56%	33%	11%	9
Clinician (or representative organisation)	0%	0%	0%	25%	50%	25%	4
Consulting	-	-	-	-	-	-	0
State / Territory government	-	-	-	-	-	-	0
Other	0%	0%	40%	40%	20%	0%	5

Many stakeholder groups also believed that increased funding and dedicated centralised support to First Nations people, and those organisations representing them, was crucial to ensure their voices are authentically embedded in the HTA process.

Patients, Consumers and Representative Groups

There was again strong support from these groups for this option.

"The solution proposed is supported and likely to have a significant impact in the assessment of HTA for First Nations peoples. This will need funding to support the implementation of the additional bridging resource." (Australasian Leukaemia and Lymphoma Group)

"BCNA would like to see community-controlled health organisations engaged in the implementation stage of this recommendation." (Breast Cancer Network Australia)

"We believe a central resource is the most sustainable and equitable approach." (Rare Voices Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was broad support for this option amongst these groups. NACCHO requested more detail in regard to this option.

"The nature of this would need to be expanded upon." (NACCHO)

"Have First Nations representation at all stages of health technology assessments." – (Australasian College for Emergency Medicine

"A dedicated resource for HTA submissions and education to assist organisations representing the health outcomes of Frist Nations peoples can help address barriers that may hinder meaningful participation, such as lack of familiarity with the HTA process or resource constraints." (Australian Healthcare and Hospitals Association)

"The dedicated resource must also review all medicines currently listed on the PBS that present an unacceptable high risk over any benefit." (Royal Australian College of Surgeons)

Table 10. 1.4. State and territory government collaboration in HTA: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	65%	29%	0%	6%	17
Pharmaceutical / Medical technology company	0%	22%	61%	11%	6%	18
University or research sector	0%	50%	0%	0%	50%	2
Industry association / Peak body	0%	50%	50%	0%	0%	8
Clinician (or representative organisation)	0%	0%	100%	0%	0%	1
Consulting	0%	0%	0%	0%	100%	1
State / Territory government	0%	0%	67%	0%	33%	3
Other	0%	40%	40%	0%	20%	5

The submissions were again very supportive of this section of the paper. There was a belief expressed that increased and formal collaboration with State and Territory governments would assist with managing funding pathways and allow for better information sharing. The potential for a centralised data sharing system was supported broadly. The variations across States and Territories, as well as the need to negotiate with buyers individually within some jurisdictions, was raised as a concern that is not directly solved though these options. The need for a federally funded system for those therapies out of scope for PBS listing was also highlighted.

Patients, Consumers and Representative Groups

Broad support from these groups was noted here. There was a comment that these options do not fully address the implementation of decisions and that this needs to be explicitly addressed.

The Collaborative Consumer Group Response representing the views of many consumers commented that this option should have "mandated timeframes from approval to access between Commonwealth and States that address the current barriers to consistent and equitable access to approved health technologies across Australia. Commonwealth and States are aligned and not competitive." (Collaborative Consumer Group Response)

"This option seems positive in that it stresses the importance of streamlined collaboration between the state and territory governments to ensure efficient decision-making processes. The proposed changes could potentially yield positive outcomes by improving timeliness, efficiency, and responsiveness within HTA processes. However, the options paper is unclear on how this will be implemented. More details are essential to ensure that the intended benefits are realised without compromising the effectiveness of the HTA procedures. Timely and equitable access to health technologies are major priorities, and the reform should ensure that these should be at the forefront of all considerations and changes." (Mito Foundation)

"The options do not fully address the implementation of decisions. This needs to be explicitly addressed to ensure that all jurisdictions are committed to HTA decisions that can be equitably implemented." (Genetic Support Network of Victoria)

Pharmaceutical / Medical Technology Companies

These companies were supportive but believed the options did not address all of the issues, the bureaucratic processes were seen as a huge hindrance to fast tracking implementation and that the lag time in being able to implement reforms could result in these options not solving the problem.

"We acknowledge the intent in section 1.4 to enable timelines and equitable adoption of new therapies funded through the NHRA, however the proposal lacks awareness of the current situation and ambition for genuine earliest possible access. Even with a positive MSAC recommendation, we must wait 6-8 weeks to receive a Public Summary Document. Only then can we enter into a price negotiation with the Commonwealth, a deed of agreement can take several more weeks to be sent to us by the Dept of Health, and supply arrangement discussions with the states or specific treatment centres can comment once price negotiations have completed. Any framework to speed patient access must overcome these open-ended timeframes." (Gilead)

"This section addresses some of the issues in relation to the relationship between state and federal funding however, a federally funded scheme for these therapies which do not fit within the purview of the PBS is required and would reduce the need for negotiation and increase the time to access for patients." (Novartis Australia)

Peak Bodies, Clinician/Researchers, Consultants, Not-For-Profits (NFPs)

There was support for State and Territory collaboration with the HTA by these groups and it was hoped this could assist with State-based delays, variations and inequity to access to best practice care. Clarity and detail were also requested as to how this collaboration could achieve better patient outcomes.

"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." (Omico)

"States are not cooperating with the Australian Government , if so not apparent. The roll out of programs is diminished through federal program being different in each state, yet the vaccine providers get their information from either state of federal, often conflicting. Consumers then lose confidence because they see 'bickering" and conflicts." (Immunisation Coalition)

State and Territory Governments / Departments

State and territory government stakeholders indicated that they would need to work through the detail of this proposed reform before they could appropriately asses its impact.

"The degree to which the issues will be addressed will depend on which HTA decisions are decided to have a significant financial or operational impact on States and Territories or defined as potentially disruptive. In particular, screening interventions especially large-scale or population-scale interventions should be included in the scope, as should genomic tests. Newborn bloodspot screening (NBS) is a useful case study given the recent process developed for decision-making on new screening tests/target conditions for the programs." (Department of Health, Western Australia)

Further to this, the Department of Health, Western Australia commented that "*State and Territory Health Departments have not been privy to the deliberations by MSAC, but the Commonwealth Department of Health and Aged Care has. This is despite all health ministers having ultimate decision-making authority for these programs (not just the Federal Health Minister). Enabling States and Territories to observe the MSAC process would improve decision-making confidence and reduce duplication of work. This would be equally beneficial for other population screening interventions, which are likely to have significant impacts on States and Territories. For example, States and Territories were also not directly engaged in the MSAC review of lung cancer screening."* (Department of Health, Western Australia)

Table 11. Development of central standardised data sharing system for utilisation and outcome data – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	44%	50%	6%	16
Pharmaceutical / Medical technology company	0%	0%	17%	78%	6%	0%	18
University or research sector	0%	0%	0%	0%	0%	100%	1
Industry association / Peak body	0%	0%	13%	63%	25%	0%	8
Clinician (or representative organisation)	0%	0%	0%	0%	100%	0%	1
Consulting	0%	0%	0%	0%	100%	0%	2
State / Territory government	0%	0%	67%	0%	0%	33%	3
Other	0%	0%	20%	40%	20%	20%	5

Many stakeholder groups called out the need for better data sharing across the Australian healthcare system. There was support for a central data-sharing system but with some caveats to protect data privacy and to provide incentives for organisations to share data. A number of groups did request assurances that there was not going to be duplication, as work in multiple areas seems to have already been progressed to achieve this. It was believed widely that better data-sharing had the potential to contribute to faster and more efficient HTA submission processes.

Patients, Consumers and Representative Groups

The need to significantly improve data-sharing was highlighted by many of these groups.

"There is a need to significantly improve the relationship between Federal and State governments/regulators (possibly through the National Health Reform Agreement (NHRA)) to ensure there is more clarity around improved data sharing arrangements and speeding up/alignment of approval processes and funding arrangements, especially the funding of specialised therapies." (Neurological Alliance Australia)

"Unified systems of collaboration to reduce gaps and meet the needs of all nationally. Data sharing across States and Federally needs to be transparent and easily accessible and understood by and for all stakeholders." (NeuroEndocrine Cancer Australia)

"Some aspects of centralised data sharing are already underway through the ARDC HESANDA program, and this should be considered prior to recommendations on this matter. Outcome data registries some incentives to support health care professionals/services to participate and contribute to such data registries would be beneficial in order to enable the resources to better capture data from centre who are delivering the care and monitoring the patient outcomes. This program of work would be strengthened through mandatory reporting of highly specialized therapeutics in clinical use. The ANZTCT Registry (formerly ABMTRR) is a good example of a mandated register to follow CAR T-cell recipients as a national effort federally funded so that all jurisdictions involved in CAR T-cell treatment can report cases for long term clinical outcome." (Australasian Leukaemia and Lymphoma Group)

"BCNA full supports this recommendation and is acutely aware of specific data sharing issues in cancer control. We would want to see these recommendations work in awareness of and collaboration with other work in this area such as the development of cancer data frameworks as part of the Australian Cancer Plan, and work being undertaken by the Australian Digital Health Agency to full leverage all opportunities to increase data sharing. Most importantly, BCNA asserts that health data in Australia must be framed as an asset to be used as opposed to a risk to be managed." (Breast Cancer Network Australia)

"CHF understands the potential benefit to consumers of central standardises data sharing but is also aware of some of the risks involved. Consumers are concerned about privacy and data guardianship. When consumer-generated evidence is to be used more consistently, adequate resources must be put in place to guarantee the establishment of strong systems that protect and maintain such data. This will lead to a virtuous cycle in which consumers are confident releasing data, leading to a richer, more fit-for-purpose database. Measures should also be put in place to prevent consumer-generated data to be used for financial gain. Consumers are adamant that while they are happy to release data for altruistic purposes, its use for financial

profit is completely unacceptable. Legislators must not shy away from the challenges of ensuring that there are clauses in place preventing this from happening." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

Whilst there was general support for these options from these companies, they did raise questions about the types of data that would be housed in the system and how it would influence decision making. They also emphasised the need for industry consultation on the development of the system. They emphasised the need for all States and Territories to be on board with this option.

"CSL supports state and territory government collaboration with federal HTA agencies to centralise and facilitate the sharing of utilisation and outcome data, and believes that this will benefit the Commonwealth, patients, clinicians and sponsors. An immediate option for reform could be the linkage of the Australian Immunisation Register (AIR) to hospital admission data, which would allow monitoring of vaccine effectiveness in real time. "Proposed initiatives to centralise sharing of utilisation and outcome data must be developed in consultation with industry, to ensure that the data collected are useful, informative and fit for purpose." (CSL Limited)

"In principle this is very welcomed to have a centralised database to facilitate sharing and standardised data collection. Some concerns are still evident, how would this be facilitated, how long would it take to implement, how would the data be used in decision making and what weighting in decision making would it be? Another concern is determining what variables are being collected and is this proactive or retrospective. Proactive data collection may in fact delay access to medicines even longer and retrospective review of outcomes - does this mean all drugs listed would be subject to a review and in that context what does that actually mean? Also, we need the ability to change variables over time in order to continue to be fit for purpose." (Antengene)

"Without agreement from all States and Territories, this option could increase the level of detail required for an HTA submission, especially if there are unique state requirements." (Boehringer Ingelheim)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was strong support from these groups for this option.

"IQVIA strongly agrees with the need to invest in improving data infrastructure and access in Australia. A wide range of valuable Real World datasets exist today, but they are often difficult to access due to long, complex and unclear approval processes. Additionally, Australia's RWD landscape is highly fragmented, with different datasets (covering different aspects of the patient care experience) governed by disparate data custodians. This makes it difficult to create a comprehensive picture of patients overall health resource utilisation and outcomes. Interestingly, we frequently encounter research questions from industry that could be well-answered via a combination of existing datasets (e.g., PBS, MBS, admitted patient data collections, birth & death data, etc.) but often these studies do not move beyond concept development due to the data being too difficult and time-/resource-intensive to access and link. Improved mechanisms for

data sharing and access would consequently help increase both the quantity and quality of evidence generated and enable more informed data-driven decision-making by all parties." (IQVIA)

"This is welcome especially for better generation of RWD. The data needs to be accessible to industry and other stakeholders making submissions." (Medical Technology Association of Australia)

"SHPA is pleased with the uptake of our recommendation to develop a repository of non-PBS, off-label and Special Access Scheme (SAS) medicines data gathered from all hospitals across Australia to facilitate more timely decision making and provide Australians with early access to medicines needed in the acute care setting. As the TGA is currently undertaking parallel consultations to inform the repurposing of medicines in Australia, SHPA believes these two areas of work should work together and further achieve collaboration and breaking down of silos in our healthcare system when it comes to medicines regulation and funding. The development of this data sharing system would be a useful resource to leverage off the experience of specialist clinicians and pharmacists, and a means of scanning the horizon for medicines commonly used in the acute care setting, to be considered for approval in Australia". (SHPA)

Table 12. Increase opportunities for consultation and work sharing – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	6%	38%	50%	6%	16
Pharmaceutical / Medical technology company	0%	6%	22%	67%	0%	6%	18
University or research sector	0%	0%	0%	0%	0%	100%	1
Industry association / Peak body	0%	0%	13%	63%	25%	0%	8
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	1
Consulting	0%	0%	0%	0%	0%	100%	1
State / Territory government	0%	0%	67%	33%	0%	0%	3
Other	0%	0%	0%	60%	20%	20%	5

Increased consultation and work-sharing is again seen by the majority of stakeholder groups as a very welcome and beneficial option. Most peak bodies and patient and consumer groups see that immense benefit could come from increased sharing of knowledge, including expertise utilisation and consistent and equitable access across Australia. A number of Pharmaceutical / Medical Technology Companies called for industry/sponsor involvement in consultation and one company believed that HTA should be done at the Federal level with a single Federal payer for therapies, as such they did not consider the above option worthwhile.

Patients, Consumers and Representative Groups

This reform was strongly supported among patient and consumer groups.

"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." (Rare Voices Australia)

"CHF support an increase of opportunities to provide input for state and territory governments, across the whole health technology lifecycle." (Consumer Health Forum)

"A great strength of the National Framework is that it fosters collaboration, partnerships, sharing of knowledge and expertise very efficiently and effectively. There is a long history of stakeholder partnership and collaboration between patients, clinicians, governments and industry stakeholders, with recognition that each contribute to best practice health outcomes and cost management. The specialists who provide treatment and care in HTCs develop and use best practice evidence-based clinical guidelines consistent with international guidelines and participate in international research. This involves both haematologists and other disciplines in the multidisciplinary HTC team, such as nursing, physiotherapy, psychosocial care and laboratory science. Likewise, the patient organisation, Haemophilia Foundation Australia (HFA), contributes to this from a consumer perspective." (Haemophilia Foundation of Australia)

Pharmaceutical / Medical Technology Companies

Strong support from these companies with requests for more detail on the implementation of this and sponsor involvement.

"More details are needed on the opportunities for work sharing by state and territory governments. For instance, there is a need to qualify when those collaborations will occur and what are HTA decisions that will have significant financial and operational impact." (Illumina)

"Inclusion of Sponsors within any consultation and work sharing with Federal and State and Territory governments for health technologies which are being evaluated through NHRA and for those outside the NHRA process needs to be included in any proposal." (Novartis Australia)

Peak Bodies, Clinician/Researchers, Consultants, Not-For-Profits (NFPs)

Strong support for this option from these groups. There was a request for hospital input into PBS indications for conditions.

"We support State and Territory collaborations in addition to national approaches, to enhance the visibility of decision-making and utilise expertise from the jurisdictions, transparent consultation, implementation planning - these issues don't just pertain to hospitals but also impact on our broader health system (e.g. the ACCHO sector)." (NACCHO)

"Once again, SHPA is pleased with this option to increase opportunities for consultation and work sharing by state and territory governments across the health technology lifecycle. However, SHPA recommends that hospitals should also be engaged and offered an opportunity to provide clinical input into PBS indications for conditions, given the extensive off-label use of medicines that is pertinent to medicines and technology regulation and funding, and has demonstrable impacts on patient access that can amount to a postcode lottery." (Society of Hospital Pharmacists)

"In theory this is good but the consultation process must not sacrifice speed to access." (Medical Technology Association of Australia)

State and Territory Governments / Departments

State and territory government stakeholders noted this reform has potential to reduce duplication. However, they also suggested a need for mechanisms to ensure that collective state & territory feedback holds equivalent weighting to previous individual feedback submissions and also captures those jurisdictions with very different requirements and available resources.

Table 13. 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) – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	6%	31%	50%	13%	16
Pharmaceutical / Medical technology company	0%	17%	28%	50%	0%	6%	18
University or research sector	0%	0%	0%	0%	0%	100%	1
Industry association / Peak body	0%	0%	13%	63%	25%	0%	8
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	1
Consulting	0%	0%	0%	0%	0%	100%	1
State / Territory government	67%	0%	0%	0%	33%	0%	3
Other	0%	0%	60%	20%	0%	20%	5

There was commentary from many stakeholder groups in regard to these options. Both Pharmaceutical / Medical Technology Companies and patient and consumer groups generally supported jointly funded technologies (such as HSTs) and believed that the impacts of this option would be very positive. They believed it could provide national cohesion and improve equitable access to consumers across the country. One pharmaceutical company and peak body believed that to further strengthen this, the joint Federal/State funding model could be removed to make way for a single Federally funded model with a centralised HTA.

The States and Territories provided some insights into the challenges they have faced implementing the current model. They also highlighted that there is already a framework in place to manage these technologies nationally, and that a cohesive approach may be difficult to implement due to the differences in the healthcare systems of the jurisdictions.

Patients, Consumers and Representative Groups

There was general support for this option from these groups, with many providing comments through their own consumers or patient lens.

"It would be very helpful to have jointly funded health technologies by the Commonwealth and state and territory governments, especially for HSTs. This would enable cohesion in the implementation and equitable access to all consumers. However, more detail is required to outline how this model will be effective." (Cystic Fibrosis)

"We welcome the development and implementation of a nationally cohesive approach to HTA as an opportunity to have consistent principles built into the approach across health technologies that are jointly funded by Commonwealth and state/territory governments. However, this needs to support and enhance rather than weaken or replace the existing National Blood Arrangements and National Framework to Manage the Treatment and Care of Bleeding Disorders." (Haemophilia Foundation Australia)

"BCNA supports this recommendation in principle but would want to ensure that further disparities between States and Territories are not created through partnerships only with specific jurisdictions and the Commonwealth." (Breast Cancer Network Australia)

"Many costly gene therapies are coming down the pipeline for rare genetic diseases like childhood dementia disorders, so funding models that share the cost and make them equitably available to all patients across the country are essential." (Childhood Dementia Initiative)

"*This should be considered a high priority to prevent people waiting for access to approved therapies."* (Lung Foundation)

"CHF supports the reform towards a nationally cohesive approach to HTA. CHF also supports the establishment of timeframes for the accelerated processing of high-cost, highly specialised therapies, provided that it does not pose unacceptable safety risks to consumers. CHF also supports the establishment of horizon scanning to facilitate timely planning and preparation for adoption by jurisdictions." (Consumers Health Forum)

Pharmaceutical / Medical Technology Companies

Supported by these companies in principle, but with several companies calling for a Federally-funded, centralised model.

"BMSA supports in principle the options presented to address issues relating to the HTA assessment and provision of highly specialised therapies (HSTs) that may be jointly funded by the commonwealth and the States and Territories. We encourage all jurisdictions to complete the work identified in Schedule C of the Addendum to National Health Reform Agreement 2020- 25 to implement financing system that is proactive, value-based and focused on individual and

community needs as soon as practicable. This should support a framework for the appropriate funding of HSTs and clarify the costs for all parties. A full, transparent and agreed understanding of the costs for jurisdictions will reduce delays in access by patients to HSTs. This company also recognised "the important role horizon scanning can play in helping jurisdictions prepare for the introduction of innovative medicines, particularly from a budget perspective. We support the options proposed in this section and in 5.2 of the options paper. Any new national approach to the assessment and funding of innovative medicines must maintain equity of access for patients. In particular, the options relating to consultation, data and work sharing must not delay unreasonably patient access to treatment." (Bristol Myers Squibb Australia)

"We fully support a nationally cohesive approach to HTA for technologies. While the proposed options seem to work towards this goal, the proposals could be strengthened by removing the Federal/State Government co-funded model to have sole Commonwealth funding, with centralised HTA. This would ensure equity between Australia's States and Territories, rather than 'postcode lotteries'. Collaboration with States and Territories will continue to be important for these products in order to ensure timely and coordinated implementation" (UCB Australia)

"While separate from the Terms of Reference for this review, differences in funding mechanisms among cell and gene therapies are driving an inequity in patient access. This could be addressed by allocating 100% of funding from the Commonwealth for all HSTs in the next NHRA. By doing so, this would reduce the requirement for State and Territory Governments to absorb the cost of HSTs within existing hospital and state health budgetary expenditure. There are opportunities to use existing reimbursement models (i.e. sponsor and Commonwealth price and risk-share arrangements) and data infrastructure which currently apply to the PBS, that could reduce contractual requirements pertaining to the cost of the HST. This would simplify the application and negotiation framework, with the State and Territories still equitably contributing to support patient access through the provision of infrastructure and the workforce needed to deliver these treatments. Prioritising the actions from the National Health Reform Agreement Addendum (NHRA) is critical to improved inter-governmental collaboration." (Roche Products)

This company also went on to explain *"the current pathway for Highly Specialised Therapies (HSTs) is challenging for governments, consumers and sponsors. The geographical inequity and delays in patient access to new HSTs, as well as the funding arrangement complexities must be addressed via the NHRA process as a priority. As noted earlier, this may ease State and Territory Government budget pressures to cover costs for treatments in the short time and delineate between issues pertaining to the value of the HST (i.e. cost of the HST), and the funding and valuation behind the implementation, including administration of the HST and subsequent patient monitoring. State/Territory input is important for informing the broader value discussion and to identify system (i.e. infrastructure/workforce) implications, however, this must be conducted in a manner that does not prolong the HTA process, and further delay patient access. Within the scope of the review, inequities across States and Territories would be partly addressed by these measures particularly (2) establishing timeframes for the implementation of HSTs and (6) initial implementation planning when combined with horizon scanning, which can be shared with, rather than conducted by, the States and Territories."* (Roche Products)

Patient access could be enhanced and efficiencies could be gained if a body like the PBAC determines a therapy as cost effective, allowing the states to implement it without additional evaluations. (Boehringer Ingelheim)

Peak Bodies, Clinician/Researchers, Consultants, Not-For-Profits (NFPs)

There was support for this option, with similar calls for a fully federally funded and centralised model.

"SHPA strongly advocates for a nationally cohesive, efficient, and responsive HTA framework to inform government investment and disinvestment decisions in Australia. HTAs must consider the broader implications of a health technology on the health system and fund the whole cost of therapy, not just the individual health technology, if we are to ensure person-centred and equitable access to health technologies, as outlined in the National Medicines Policy (NMP). The current lack of suitable funding pathways that provide subsidy for the whole cost of therapy results in inequity in access and creates perverse incentives, ultimately impacting on consumer health outcomes and further costing the health system." (Society of Pharmacists of Australia)

"Recognition that HTA applies to patients regardless of where they live and regardless of which funding source is utilised (State vs Federal) is very important. Early engagement with state and territory authorities in collaboration with the federal funding pathways and recognition of the overall health economy as it pertains to an individual patient is an important step to assessing the relative value of new treatments." (Clinician)

State and Territory Governments / Departments

State and territory stakeholders tended to provide more nuanced feedback give their significant familiarity with current co-funding models.

"For high cost, highly specialised therapies greater consultation and engagement with state and territory governments is valuable before proceeding to implementation as the costs are shared between the Commonwealth and State/Territory governments. In situations where there is a lack of consensus between the Commonwealth and/or relevant HTA Committee and State/Territory governments there should be a mechanism where the advice/input from all relevant parties is considered and provided to the Minister as part of the Ministerial Recommendation decision making." (ACT Health)

"MSAC reviews of conditions/tests for newborn bloodspot screening programs has highlighted a difficulty relating to how States and Territories should be engaged and how costs to States and Territories are captured. Implementation planning may not be possible prior to or during the HTA if multiple implementation models are being considered and it is not yet known which model will be recommended. Providing implementation advice on a broad range of possible models is resource intensive for States and Territories, but it is important that downstream impacts on State and Territory health systems are incorporated. Further work on how to collect and incorporate this information would be welcomed." (Department of Health, Western Australia)

One government stakeholder noted that their devolved health-delivery model means price negotiation with suppliers needs to occur at the entity level, which can be another factor in delaying access to patients.

Section 2: Health technology funding and assessment pathways

Stakeholders were invited to provide written comment on the reform options presented for health technology funding and assessment pathways as per the table below (reproduced from the HTA Review's Options Paper).

Subject	Key option/s
2. Health technology funding and assessment pathways	
2.1. Streamlining and aligning HTA pathways and advisory committees - Overarching goal: a staged approach (including short, medium and longer-term steps) to achieving a simplified (single entry) HTA gateway reflecting nationally consistent HTA approach.	
Pathway for drugs for ultra-rare diseases (Life Saving Drugs Program (LSDP))	1. Develop and publish a Statement of rationale for the LSDP outlining principles underpinning the program, and the eligibility criteria, including the value-for-money consideration by reference to the overarching recommendations of the LSDP Review Expert Panel recommendation. 2. PBAC to become the sole HTA committee for drugs for ultra-rare diseases to eliminate double handling. The expertise on the LSDP expert panel will inform and support decisions regarding therapies for ultra-rare diseases. 3. PBAC advises the Minister on key requirements to enable listing on the LSDP based on a comparative assessment of effectiveness and cost.
Vaccine pathway	1. Streamline the pathway for listing of a vaccine on the National Immunisation Program (NIP) by removing the requirement for the sponsor to get Australian Technical Advisory Group on Immunisation (ATAGI) advice prior to submission. The revised process would be as follows. 1. The sponsor of a vaccine makes a submission to the PBAC for the NIP 2. The PBAC evaluators and vaccine evaluation experts evaluate the sponsors submission and produce a single comprehensive assessment report 3. The PBAC Economic Sub-Committee (ESC) is supplemented by the appropriate ATAGI representatives (specialists for particular type of vaccine and disease) to provide formal (ESC + ATAGI) advice to PBAC 4. PBAC provides advice and recommendation to government on the clinical and cost effectiveness of the vaccine for the NIP. *Note: A review of the NIP is underway and it is expected this will include consideration of the procurement process and strategies to better coordinate and streamline the procurement and implementation of vaccines.* 2. Horizon scanning for vaccines is established including appropriate stakeholders to ensure that ATAGI can be prepared to provide advice. 3. Develop a mechanism and criteria to have the assessment of vaccines be proportionate to the level of risk of the product.

	Note*: These changes are not intended to preclude the ability for sponsors to seek early advice from ATAGI or modify/remove any of functions of ATAGI.*
Expanding role of PBAC	a. Further expanding the advisory role of the PBAC to enable it to make the HTA recommendation to the Minister for Health and Aged Care for a broader range of health technologies including codependent health technologies. (short term) b. The HTA advice does not presume all subsequent funding decisions would take effect through the PBS.
Unified HTA pathway for all health technologies with Commonwealth funding	Develop a unified, national, HTA pathway for all health technology evaluation (medium to long-term) 1. To meet this aim, investigate approaches for having one committee* that is appropriately resourced (including adjustments to Committee composition and scope) that could progress all HTA by drawing on pools of appropriate specialists as needed, including for medicines, advanced therapies, blood and blood products and other types of technologies seeking public funding. 2. The Committee responsible for assessing a submission should have the flexibility to recommend the most suitable funding pathway for that product. 3. It is noted that the committee structure may need to be augmented to ensure that it appropriately resourced both with expertise and workload. 4. The HTA advice does not presume all subsequent funding decisions would take effect through the PBS. *The goal of this is to have a unified HTA committee approach however with respect to workload, this could be done through more frequent meetings or having multiple committees with a unified approach and offset meeting cycles. Additionally, the committee expertise could be augmented through additional permanent members, having topic specific groups that can be drawn on to provide advice, or pools of topic specific experts that can be drawn on to supplementary members as the expertise is required.

2.2. Proportionate appraisal pathways: Development of pathways to calibrate the level of appraisal required for HTA submissions to the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents.

Triaging submissions	As a part of both the proportionate appraisal and streamlining of HTA pathways and committees, HTA submissions for Australian Government Subsidy should utilise a 'single front door' approach so submissions may be triaged to determine the appropriate evaluation and appraisal mechanisms. The triaging stage would determine: 1. the appropriate appraisal pathway for the HTA submission (based on risk and other factors) 2. appropriate constitution/membership required for HTA committee and technical sub-committees based on type of technology and other factors (e.g. for consideration of vaccines or specific diagnostic tests) 3. the PICO scoping/consultation/confirmation required 4. the meeting date for the HTA consideration (based on the above). While the decision of the appropriate HTA pathway would be through consideration of a triaging body (could be similar to PBAC Executive or other constituted triaging body), the development of a clear and transparent decision tool such as a decision tree would improve consistency, reduce workload, and help support fit-for-purpose submissions.
Streamlined pathway for cost-minimisation submissions (therapies not claiming a significant improvement in health outcomes or reduction in toxicity)	1 Develop criteria for therapies to be eligible for streamlined cost-minimisation pathway. 2 Submissions for therapies not claiming a significant improvement in health outcomes, would undergo an abbreviated evaluation and consideration by the ESC; if it can be determined that the therapy meets the developed criteria it would be fast tracked to the price agreement stage after out-of-session consideration by the PBAC Executive (or similar). 3 Information regarding the price of the comparator the proposed therapy is cost-minimised against would be shared with the sponsor early in the process prior to HTA committee consideration. This would allow sponsors to make an informed decision regarding whether to proceed or withdraw the submission from consideration (if the potential pricing outcome is not within sponsor expectations). Withdrawal of submissions that would unlikely proceed to implementation following a positive HTA committee recommendation may prevent the unnecessary use of valuable HTA evaluation and administrative resources. 4 For submissions that do not meet the developed criteria, the PBAC executive can nominate for the submission to either be considered without change by the PBAC in the current cycle or the next cycle, allowing the sponsor time to address issues raised, noting the sponsor would have the discretion to withdraw their submission.
Early resolution mechanisms for submissions of	For health technologies that are comparatively clinically safe and effective and represent HATV in an area of HUCN (where submission meets set criteria), but where there is uncertainty related to the economic model or the price.

major new therapeutic advances in areas of HUCN	<u>Criteria:</u> a. Therapies that offer likely HATV in areas where there is HUCN, and b. Submission made to the PBAC at the same time as TGA application is made, or at the earliest opportunity after TGA application is made, and c. Submission lodged within 6 months of receiving first regulatory approval from a comparable overseas regulator (e.g. Food and Drug Administration (FDA)/European Medicines Agency (EMA)) <u>Alternative option 1: Introducing an optional resolution step **before** HTA committee consideration:</u> a. After the submission has been evaluated and considered by PBAC Economic Sub-Committee (ESC), sponsors would be provided with preliminary advice on their submission, and the option to either: 1. progress submission to HTA Committee for consideration 'as-is' (with risk of negative recommendation and exit from the HTA cycle); or 2. undertake a resolution process to address identified deficiencies/technical concerns under a set/time-limited period (e.g. up to maximum of one HTA cycle length of ~17 weeks), before progressing to HTA Committee consideration. b. With this approach, the relevant ESC discussants, evaluators, Departmental staff and the sponsor would meet and work iteratively towards addressing deficiencies/technical concerns with ESC advice, prior to a PBAC consideration so that it is more likely to receive a positive recommendation. c. After the resolution process, the submission would go to PBAC where a recommendation to the minister would be made regarding the listing. d. This would be a time limited process running contemporaneously to the TGA assessment, resulting in expeditated access. e. It is intended that the optional early resolution process will avoid a negative recommendation, however in the rare occasion where the application is not recommended, there would be a restriction on the ability for sponsors to re-submit. f. Note: Understanding feasible and practical solutions / strategies to reduce the number of resubmissions under these options is a key detail to workshop with stakeholders through this consultation process. This could include setting a maximum allowable number of submission (e.g. only 1 resubmission allowed). **<u>OR</u>** <u>Alternative option 2: Introducing an optional resolution step **before** HTA committee consideration, with additional post committee resolution:</u> 1. As above in Alternative option 1, for points 1 - 4 2. Point 5 above in Alternative option 1 would change to include, where an application is not recommended, the sponsor and the Department will meet to determine future opportunities for resolution and criteria for future submissions. **<u>OR</u>** <u>Alternative option 3: Early Price negotiation</u>

	• As above in Alternative Option 1, however pricing negotiation would (optionally) occur after the provision of early PBAC ESC advice, prior to HTA Committee consideration. In order to provide greater certainty to PBAC and provide the ability to recommend/not recommend at the negotiated price, price negotiation could be included earlier in the evaluation cycle. Advice from the ESC would more actively indicate to sponsors and the Department that the product is unlikely to be considered cost-effective at the proposed price; this would serve as a trigger for price negotiations to be conducted concurrently. Additionally, as the negotiated price would be included in the economic model at the time of consideration PBAC can have greater certainty in its decision-making. **OR** Alternative option 4: Introducing an optional resolution step **after** HTA committee consideration but **before** advice is finalised a. After the HTA committee has considered the submission, the sponsor is provided information on a provisional negative recommendation by the HTA committee and the option to either: 1. undertake a resolution process to address identified deficiencies/technical concerns under a set/time-limited period (e.g. up to maximum of one HTA cycle length of ~17 weeks), before progressing to the HTA Committee for a second consideration, or 2. agree to ratify the negative recommendation and exit the HTA cycle. b. With this approach, the relevant advisory committee members, evaluators, Departmental staff and the sponsor would meet and work towards addressing deficiencies / technical concerns. c. Following the resolution process, the submission would go to the HTA committee where a recommendation to the Minister would be made regarding the listing. d. This would be a time limited process running contemporaneously with the TGA assessment, resulting in expedited access. **e.** If the application is not recommended the second time it is considered by the HTA committee, there will be no immediate opportunities to submit revisions and the sponsor and the Department will meet to determine future opportunities for independent arbitration, and criteria for future submissions.
Expanding resolution step to all relevant cost effectiveness submissions	After piloting with therapies with HATV in areas of HUCN the early resolution step could be expanded to other relevant cost effectiveness submissions.
Development of a disease specific common model (reference case) for disease areas with	Develop and adopt a consistent model structure for specified disease areas where there are many potential therapies / technologies under development (as identified through horizon scanning). This should include input from a wide range of stakeholders to ensure a comprehensive representation of the disease area. Disease specific models would include outlining the analytic methods, the model structure, and some parameters. This would enhance consistency in decision-

high active product development	making through increased comparability of models across different technologies for the same disease/condition. As the development of disease--specific models would require significant investment to develop, they would only be used for disease areas where many subsequent submissions would utilise the model. Additionally, further investment will be required to maintain the models over time to ensure they are current and relevant for the treatments and disease pathways for which they are intended. These models will also enable re-assessment of health technologies (post market review) after PBS listing. Australia should investigate international collaboration on the development of disease-specific common models.
Decouple the requirement for the TGA Delegate's overview to support PBAC advice	Enable full parallel processing of TGA and PBAC submissions by enabling the PBAC to communicate its likely advice to sponsors prior to receiving the TGA delegate's overview. The PBAC's final advice to Government, and resulting funding arrangements, would still be required to be consistent with the TGA delegate's overview and Australian Register of Therapeutic Goods (ARTG) listing.
Case manager	Resourcing to support allocation of a case manager to facilitate communication and information sharing between the Department and applicant for cost-utility analysis (CUA)/ cost effectiveness analysis (CEA) applications. This is to be modelled off the current case management approach used for positive PBAC recommendations that progress through pricing pathway A. Submissions would be assigned a case manager from their notice of intent to make a submission.

Section 2 – Overall summary

Overall, there was broad "in-principle" support for the options outlined in 2.1 and 2.2 of the Options Paper, and an acknowledgment from stakeholders of the opportunities that streamlining pathways and consolidating committees presented in achieving efficiencies in the HTA timelines and processes. Key concerns raised centred on the resource load this would place on the unified process and the challenges of one pathway having the requisite breadth of knowledge to effectively and assess the diversity of both current and emerging technologies (especially gene therapies, which are noted as a key omission from the reform options presented).

There was strong rejection across the pharmaceutical industry of a trade-off between price and an abridged/shortened HTA assessment process for cost-minimisation submissions. The proposed criteria of certain submissions needing to be lodged within six (6) months of receiving first regulatory approval from a comparable overseas regulator was also viewed as too restrictive.

In terms of the alternative options put forward for early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN, consumer groups commonly said that earlier engagement and resolution of issues was seen as offering greatest scope to reduce HTA delays. They tended to favour those encompassing earlier engagement (Options 1-3 in the Options Paper) – albeit with a view that price issues should only be considered as secondary to safety, efficacy and equity considerations.

Pharmaceutical and other stakeholders generally favoured Option 4 in the Options Paper given this provides greater certainty post evaluation – however several stakeholders queried how this option was vastly different to the current HTA process. A key issue of concern across the Options is the proposal to limit number of resubmissions, which was identified as potentially resulting in fewer products being brought to market.

The concept of disease-specific models was supported by consumer stakeholders (pending appropriate input into their development) but were viewed as less useful among other stakeholder groups, some of whom pointed at the limited success of such models in other jurisdictions.

2.1 Streamlining and aligning HTA pathways and advisory committees

Table 14. 2.1. Streamlining and aligning HTA pathways and advisory committees: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	57%	17%	0%	26%	23
Pharmaceutical / Medical technology company	0%	15%	65%	10%	10%	20
University or research sector	20%	20%	20%	20%	20%	5
Industry association / Peak body	0%	30%	30%	10%	30%	10
Clinician (or representative organisation)	0%	40%	40%	0%	20%	5
Consulting	0%	0%	67%	33%	0%	3
State / Territory government	0%	0%	0%	100%	0%	2
Other	0%	20%	20%	40%	20%	5

Patients, Consumers and Representative Groups

Those patient or consumer groups most supportive of the streamlining and aligning of HTA pathways indicated that this reform – if implemented in an appropriately resourced and considered manner – could potentially increase the timeliness of HTA assessments, and reduce time to access for patients.

"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." (Rare Voices Australia)

"MSCAN understands that the proposed options for 'early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN' will deliver expediated access but only for a small portion of medicines. We maintain that options that allow for early access for the majority of medicines are needed....the treatment paradigm has shifted tremendously for melanoma and some skin cancers as a result of medicines and we support treatment options being available for Australian patients as quickly as possible once safety and efficacy are assured. Pathways that facilitate expediated access and ensuring that medicines are accessible for Australian patients should be a priority." (Melanoma & Skin Cancer Advocacy Network (MSCAN))

"Streamlining HTA processes is welcomed to reduce the time from TGA registration to listing on the PBS. We hope that this major change will not slow down processes in the meantime and it can be implemented in a timely manner without too much disruption to getting life saving medicines to patients." (Childhood Dementia Initiative)

"A unified approach is essential. We request a simplified (single entry) HTA gateway which reflects a consistent HTA approach nationally." (NeuroEndocrine Cancer Australia)

Those providing more qualified support for this reform commonly noted the importance for stakeholder engagement via a detailed co-design stage, as well as ensuring the unified pathway accessed both the consumer voice (ideally from those with direct lived experience of the target condition) and the breadth of specialist knowledge and expertise needed to understand the specific type of technology being assessed.

"These options represent significant change. Transfer from specific pathways to a single entry will require ongoing consultation and communications with all key stakeholders, including consumers. More detail is required to understand if they can achieve their intended objectives." (Collaborative Consumer Group Response)

"Having the right expert clinicians and patients at the table with the Committee will also streamline the process. Hurdles can be managed at the time with their expertise by providing or sourcing relevant advice or data, rather than the matter under question being referred to another round of evaluation." (Haemophilia Foundation Australia

"Mito Foundation supports the measures described in these reform options that have the potential to increase timely and equitable access to health technologies for all Australians. However, an implementation plan is needed which takes the following into account:

- *Consumers are engaged throughout this process. All decisions should be made with appropriate expertise guiding decision-making. This includes rare disease expertise and consumer representatives.*
- *Strengths in existing processes are not lost; whatever changes are made still need to be fit for purpose. One example is the expertise of MSAC in making decisions about genomic testing.*
- *Clarity on the transition process of existing committee(s') functions to the PBAC and then to the HTA committee.*
- *Any changes should ensure that there are no additional barriers to access for complex, high-cost treatments for ultra-rare diseases.*
- *Ensure that the streamlined pathway doesn't instead add inefficiency to the HTA system due to the increased scope of health technologies."* (Mito Foundation)

"We recognises the need to address issues including timeliness, streamlining, and approvals that recognize the rapid evolution of these technologies and their impact on diabetes care and management However, a 'super committee' (potentially with a medicines subcommittee and/or a pharmacy subcommittee) will not necessarily streamline and align pathways – again, this is a question of the effective implementation of any reforms, and the new committee(s) improving their practices (and being properly resourced to realise these improvements)." (The Australian Diabetes Alliance)

"NAA members representing rare disease groups, including Mito Foundation, are cautious about the proposal to integrate the life-saving drug program (LSDP) decision-making process into the

PBAC process. It is essential that the existing guidelines and clinical expertise - a strength of the existing LSDP process - be retained, while still realising the timeliness benefits of a single assessment process." (Neurological Alliance Australia)

There were a number of concerns identified across the submissions from consumer and patient organisations. Several submissions suggested price needed to be afforded the lowest priority after clinical need, safety and effectiveness considerations, while others questioned whether a more streamlined process might not have sufficient rigour and thoroughness. Others noted that limiting the number of resubmissions could reduce submissions in areas of higher uncertainty.

"Consumer do not support options that use price as an entry point for ensuring timely access. HTA assessments should initially prioritise clinical need, safety and effectiveness separately to pricing negotiations/considerations." (Collaborative Consumer Group Response)

"We are concerned that streamlining may forgo detail and thoroughness needed for First Nations-related submissions, but certainly not averse to faster listing conceptually." (NACCHO

"Prescribing a limited number of resubmissions – we support all efforts to avoid multiple resubmissions as resubmissions represents a delay in access however mandating a limited number of resubmissions may have the unintended effect of disincentivising submissions where this is a high level of uncertainty" (Collaborative Consumer Group Response)

Pharmaceutical / Medical Technology Companies

Several Pharmaceutical / Medical Technology Companies indicated that a unified pathway did offer some potential to reduce complexity and ensures limited HTA resources are deployed in a manner that better reflects the risk profile of the health technology being assessed, especially cost-minimisation/non-inferiority submissions.

"ST supports any initiative to streamline HTA pathways and advisory committees. We further believe that provision should be made for direct pricing negotiations following a positive TGA assessment for orphan therapies. We would further ask the review committee to consider that when a therapy is already internationally approved and will only result in Commonwealth expenditure of less than $10M in AU, then these therapies should be subject only to a direct price negotiation. This would minimise submission churn and ensure expedited access for patients. A temporary price could be negotiated until a basket of reference country prices emerge." (Specialised Therapeutics)

"[We] support the view that the level of appraisal should be risk calibrated and be flexible and that early resolution of PBAC identified issues in submissions offers a solution to reduce the number of resubmissions. [We] believe that all submissions should be able to seek early resolution and establishing restrictions around entry to this pathway would unnecessarily limit the potential positive impact of the approach." (AstraZeneca)

As with the consumer groups, concerns around these pathway reforms for Pharmaceutical / Medical Technology Companies tended to centre around access to the right expertise and knowledge to make informed decisions on the evaluation framework, the additional resourcing that will be required to support a single pathway, and the impacts limitations on re-submissions

may have on timely access to medicines. he linking of an expedited cost-minimisation pathway to price reductions was also viewed negatively by some.

"Must ensure appropriate expertise available and sufficient capacity." (Eli Lilly Australia.)

"CSL supports the overarching goal of a "simplified single entry HTA gateway" (i.e., a single submission to PBAC for NIP listing, with ATAGI and PBAC evaluators collaborating to develop a single assessment report) but believes that the option to seek pre-submission advice from ATAGI must also be retained...It is unclear how and when during the process the PBAC and ATAGI evaluators will interact, and whether this is via individual collaboration or committee discussion to gain consensus. It is important that the opinions of the PBAC and ATAGI evaluators are able to be presented independently and given equal consideration in the evaluation process." (CSL Limited)

"Imposing limits around the number of resubmissions could have the unintended consequence of slowing time to medicines access. The linking of an abbreviated cost-minimisation pathway to price reductions would also have a very negative impact on the time to patient access, the choice of therapies available and numbers of treatments available to Australian patients." (AstraZeneca)

Others noted that they would need more detail on how this reform would be implemented at a practical level before being able to support such a measure.

"All of the suggestions presented involve the development of new processes or require investigations with little detail around what these new processes will be, the timelines involved in their development and implementation and the involvement of industry in this. As such it is not possible to support these options until additional detail is shared." (Novartis Australia)

"Based on the information provided within each of the reform options Roche believes that with the level of detail provided, it is unclear how either of the options would address the outlined issues that relate to them. To fully consider the impact on all stakeholders, further detail and development of the reform options should be shared." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

While some across these stakeholder groups were supportive of the reform option, others questioned the usefulness of the common disease reference model, the challenges of a single committee having sufficient breadth of knowledge and the resource impost of a single pathway.

"Extremely important to streamline processes and avoid current siloed assessments and duplication." (Health Services Research Association of Australia and New Zealand)

"There is benefit in have better alignment of processes for PBAC and MSAC." (Kirby Institute)

"We cannot understand how the suggested single access gateway approach can efficiently accelerate the HTA process and shorten assessment times. The vast diversity in complexity of technologies and services mitigate against such a strategy...A single gateway model would further extend meeting duration - PBAC meetings take ~3-4 days, similarly for MSAC meetings

- which leads to the question, what is the planned meeting duration to cover both PBAC and MSAC through a single gateway?" (Pathology Technology Australia)

"The impact of some of these proposals is unclear particularly on the MedTech industry. It is highly inappropriate that this foundational issue about HTA processes is being reviewed completely without MedTech (including digital health) in the terms of reference. This makes it more difficult to judge." – (Peak Body –Medical Technology Association of Australia)

One organisation also called for new pathways to be considered for high-risk populations.

"I would grateful if the Health Technology Assessment Review would consider an evidence-based, streamlined pathway to allow evolving diabetes technology, including continuous glucose monitoring, to be subsidised for high-risk populations such as Aboriginal and Torres Strait Islander Communities with type 2 diabetes. In an ideal world, this pathway would also be extended to those people with type 1 diabetes and other high-risk groups with type 2 diabetes." (Clinician - Australian Centre for Accelerating Diabetes Innovations/University of Melbourne)

Table 15. Pathway for drugs for ultra-rare diseases (Life Saving Drugs Program (LSDP)) – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	12%	41%	35%	12%	17
Pharmaceutical / Medical technology company	0%	0%	35%	45%	5%	15%	20
University or research sector	0%	0%	100%	0%	0%	0%	4
Industry association / Peak body	0%	13%	25%	25%	25%	13%	8
Clinician (or representative organisation)	0%	0%	25%	50%	0%	25%	4
Consulting	0%	0%	100%	0%	0%	0%	3
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	33%	0%	33%	33%	0%	0%	3

Most stakeholders were either positive or neutral towards this specific reform.

Patients, Consumers and Representative Groups

Those providing specific comment on this reform option were broadly supportive and suggested this offered scope to provide more timely access to life saving drugs, while some noted the importance of ensuring specialist LSDP expertise (especially clinical expertise) is maintained in any changed process.

"The unified national HTA pathway is preferred rather than piecemeal components however the options outlines under the LSDP will improve current practice" (Genetic Support Network of Victoria)

"CCA supports these options to improve the process and reduce duplication for LSDP. IBD is not an ultra-rare disease, however there is a paediatric subgroup of very young children with IBD (Very Early Onset – VEO-IBD). They are small group with high morbidity, and some mortality who would benefit from access to advanced therapies that have no other path to access." (Crohn's & Colitis Australia)

"We support a single assessment process for ultra rare disease therapeutics to speed up access, however it is important to retain existing guidelines and clinical expertise that is a strength of the existing LSDP process." (Childhood Dementia Initiative)

"It is essential that the existing guidelines and clinical expertise - a strength of the existing LSDP process - be retained, while still realising the timeliness benefits of a single assessment process." (Neurological Alliance Australia)

Others indicated that while broadly supportive of this reform, further information is needed to fully assess its possible impact on the HTA process.

"Any decisions made should be guided by appropriate expertise, including rare disease experts and consumer representatives. The inclusion of LSDP decisions into PBAC has the potential to shorten the time taken to make a decision. However, decisions must continue to be based on a distinct set of guidelines and ensure that appropriate rare disease clinical and consumer expertise. Any change made should ensure that there are no additional barriers to access for complex, high-cost treatments for ultra-rare conditions." (Mito Foundation)

"Point 2.1.1- what is the eligibility criteria when considering value for money, what price is a life? It is essential that point 2.1.2 is enacted for reducing double handling and delays." (NeuroEndocrine Cancer Australia)

Pharmaceutical / Medical Technology Companies

While many Pharmaceutical / Medical Technology Companies supported this reform in principle, many had questions about how the revised process would work in practice and wanted further clarification across a number of points.

"Biogen support the streamlined pathway for drugs for ultra-rare diseases. The PBAC advisory role to recommend listing of a medicine on the LSDP must be made to the Minister and the CMO as the Minister's delegate as in the existing framework" (Biogen)

"The LSDP is a tool for faster access for patients. Appreciate the PBAC become the sole HTA committee to make the advisement, however what is missing is the timelines of when those decisions will be made. The recommendation is positive if it reduces time to access for patients, but only if that is achieved which is not clear." (Antengene Australia)

"AZ supports this recommendation and believe that a statement of rationale for the LSDP be published that reflects broader eligibility of access to the program, along with guidance on value-

for-money consideration. The cost-effectiveness of treatments for rare diseases can be highly uncertain because of rare disease natural history data gaps, limited availability of comparator efficacy data, and small patient populations which limit the statistical analyses of clinical studies. The development of value for money criteria suggested in the Options paper should reflect the data limitations associated with rare diseases. An LSDP sub-committee should be established as part of PBAC to consider submissions. The program should remain separated from the PBS" (AstraZeneca)

"This initiative can deliver faster access by removing the need for negative PBAC recommendation on a cost effectiveness basis before a therapy is considered for inclusion on the LSDP. However, it isn't clear how this pathway will be funded. It is important that therapies for vulnerable patients with ultra-rare diseases continued to be funded without the need to demonstrate cost-effectiveness and that new pathways are adequately funded." (Pfizer)

"Roche supports, in principle, arrangements that simplify process and consolidate assessment by multiple sequential Committees. Reducing double handling, without extension of the evaluation process, and thereby accelerating access for patients, is supported in principle. Roche is supportive of a streamlined pathway for the consideration of products under the LSDP, but only on the basis that double handling is reduced, and access for patients is accelerated. Consolidation of HTA committees should not result in the removal of key programs; Roche does not support the removal of the LSDP as it remains a vital access program for patients who require life-saving treatments in rare conditions which are not considered cost-effective enough to list on the PBS. Further scoping and consultation of the PBAC's remit is required given that: "Entry to the existing LSDP pathway requires that a drug is not cost-effective but does not explicitly require consideration of value-for-money" yet the proposed Option states: "PBAC advises the Minister on key requirements to enable listing on the LSDP based on a comparative assessment of effectiveness and cost." (Roche Products)

"If implemented, the intent of the LSDP to fund drugs for ultra-rare diseases must be retained. The PBAC advisory role to recommend listing of a medicine on the LSDP must be made to the Minister and the CMO as the Minister's delegate as in the existing framework." (Biogen)

"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." -(Alexion)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholders expressed a variety of views across their comments on this specific reform.

"On the surface the incorporation of the pathway for drugs for ultra rare diseases (life-saving drugs program LSDP) into the PBAC remit may present advantages however clinicians would not want to see this process disadvantage patients in any way or slow access to life saving drugs. This system may have been set up as an alternative pathway for a reason… e.g. due to inadequacies in the PBAC process. Clinicians would need to be reassured that these issues were addressed as part of this reform. This may include acceptance that the treatment will not be supported by the same evidence as more common conditions and recognition that the therapy may not be cost effective... and meet different criteria for funding." (Clinician)

"I answered neutral because I wonder if there could be an option to make the LSDP redundant. If the expanded PBAC has the flexibility to provide value judgments on what is and isn't cost-effective, then rather than, "PBAC advises the Minister on key requirements to enable listing on the LSDP based on a comparative assessment of effectiveness and cost" (which is a form of cost effectiveness analysis by another name) could the PBAC just advise that listing on the PBS is sufficiently cost-effective in the circumstances. Thus making the LSDP funding mechanism redundant over time? The statement of rationale referred to in the options paper could apply to these specific type of PBS listings." (THEMA Consulting)

Table 16. Vaccine pathway – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	13%	31%	31%	25%	16
Pharmaceutical / Medical technology company	5%	0%	20%	55%	5%	15%	20
University or research sector	0%	0%	25%	0%	0%	75%	4
Industry association / Peak body	0%	13%	25%	25%	25%	13%	8
Clinician (or representative organisation)	0%	0%	25%	50%	0%	25%	4
Consulting	0%	0%	33%	33%	33%	0%	3
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	33%	0%	0%	33%	0%	33%	3

Patients, Consumers and Representative Groups

Those stakeholders that provided comment on this specific reform were mostly positive.

"CCA supports the expanding the role of PBAC for a broader range of health technologies including co-dependent health technologies. This will be critical for emerging cell-based therapies and potentially gene editing for monogenic causes of chronic inflammatory diseases. Cell based therapies are not 'pharmaceutical' and have no clear funding pathway." (Crohn's & Colitis Australia)

Pharmaceutical / Medical Technology Companies

The majority of Pharmaceutical / Medical Technology Companies providing feedback on this specific reform were positive in their comments, suggesting this reform does offer scope to streamline vaccine assessment pathways.

"This initiative will likely achieve much needed faster access to vaccines by removing the need for separate ATAGI advice before making a PBAC submission. It is important in developing this pathway that opportunities to seek expert advice from ATAGI remain available when needed during the process of submission development (prior to PBAC submission) to ensure important considerations such as modelling assumptions can be incorporated in the initial submission, rather than only during the evaluation process which may result in additional re-submissions to PBAC being required. Flexibility is essential, given some vaccine submissions are complex and will require detailed expert advice to align on clinical considerations for an immunisation program and PICO elements, while more simple submissions may require only limited pre-submission advice." (Pfizer)

"Roche in principle supports arrangements that simplify process and consolidate assessment by multiple sequential Committees. Reduction in Committee double handling, without extension of the Evaluation process, thereby accelerating access for patients, is supported in principle." (Roche Products)

"Biogen supports a streamlined pathway for vaccines, provided the ATAGI advice remains sufficiently broad, including advice on the program, clinical evidence and inputs to the economic model, and sufficiently robust. There will be need for transparency in how this advice was considered by the PBAC." (Biogen)

Others were more cautious in their support for this reform, with many wanting additional detail on the relationship between an expanded/single PBAC pathway and ATAGI given ATAGI's clinical expertise and experience.

"If implemented, this pathway must retain robust advice from the ATAGI to ensure that clinical issues remain at the forefront of deliberations." (A.Menarini Australia)

"The nature and timing of interactions between the PBAC and ATAGI evaluators, and the evaluators and sponsors, must be clearly defined within the new amalgamated PBAC/ATAGI process. Planned interactions must allow for more frequent opportunities for discussion and resolution of questions between the evaluators and sponsors. Experts and evaluation groups selected to evaluate vaccine submissions and provide advice to ATAGI, PBAC and ESC must have relevant expertise in the specific disease area and be highly skilled in dynamic modelling required for infectious diseases." (CSL Limited)

"While streamlining the pathway should be a positive approach, the final proposal needs to ensure that the future value of the vaccines is appropriately captured (i.e. reducing the discounting rate)." (UCB Australia)

Others did not support the proposed reform based on concerns around ATAGI being better able to understand (and value) the benefits to Australian society of specific vaccines.

"Do not progress 2.1: Vaccine pathway option 1 as proposed. Streamline process while retaining the comprehensive independent input of the Australian Technical Advisory Group on Immunisation (ATAGI), recognising the value of vaccines on patient outcomes." (GSK)

Table 17. Expanding role of PBAC – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	11%	50%	22%	17%	18
Pharmaceutical / Medical technology company	0%	19%	19%	38%	10%	14%	21
University or research sector	0%	0%	25%	25%	25%	25%	4
Industry association / Peak body	0%	11%	11%	44%	11%	22%	9
Clinician (or representative organisation)	0%	0%	0%	50%	25%	25%	4
Consulting	0%	33%	33%	33%	0%	0%	3
State / Territory government	50%	50%	0%	0%	0%	0%	2
Other	25%	0%	25%	50%	0%	0%	4

Patients, Consumers and Representative Groups

Most consumer and patient advocacy groups were supportive of this reform option, albeit with caveats of the need for adequate resourcing and also the need to manage the risk of losing specialist expertise and knowledge built within specialist sub-committee arrangements over many years.

"Consumers are generally amenable to the idea of unifying the HTA pathway for all technologies. A unified process will allow for better access to health technologies, and reduce the preventable deaths created by current barriers and inconsistencies. Despite this, consumers are also worried about the way such a process will be executed. Proper unification will require a very sizeable amount of funding and HTA structure augmentation. The risk of a half-baked unification process will ultimately borne by consumers, who will experience the loss of expertise of de- funded local HTA bodies. If this option is implemented, specialist bodies must be appropriately resourced to enable them to provide advice that is pertinent and up to date." (Consumers Health Forum of Australia)

"Under current National Blood Arrangements the process to consider new bleeding disorders therapies for funding involves several steps. Streamlining some of these arrangements through a triaging stage and a parallel process for TGA and HTA evaluation would improve the process. However, it will be imperative to accommodate processes such as tendering and not to lose the very valuable elements of the National Blood Arrangements that already exist and have shown themselves to be strategic and cost-effective over the last 25 years." (Haemophilia Foundation Australia)

"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)." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

Views on this specific reform were less uniform across Pharmaceutical / Medical Technology Companies. While many noted the capability of a unified pathway to deliver a more streamlined process, others noted the importance of ensuring PBAC's role remained in an advisory capacity only, and not for this reform to include any additional decision-making capacity. As with consumers, a number noted the need for additional resources to ensure such a change did not create a bottleneck in the assessment process.

"Any expansion to the role or remit of the PBAC must ensure that the PBAC remains advisory body only. The final decision to fund new treatments must remain with the Minister for Health and Aged Care." (A.Menarini Australia)

"AZ believe that the proposed consolidation of assessment functions could have a positive impact on improving HTA evaluation efficiency. The approach for having one committee should be investigated for co-dependant and cell and gene therapies. The pathway for vaccines could be streamlined by removing the requirement for Sponsors to receive ATAGI advice prior to submission. AZ supports the option that the PBAC Economic Sub-Committee (ESC) could be supplemented by the appropriate ATAGI representatives to provide formal (ESC + ATAGI) to provide advice to PBAC. AZ agree with the Options paper that such changes should not preclude the ability for Sponsors to seek early advice from ATAGI or remove any of functions of ATAGI. A unified approach could include a single HTA advisory committee of necessary experts, which is augmented with additional permanent members, or topic specific groups that provide advice. It would add significant workload to the Committee's already very heavy agenda. Therefore, this option would need to include increased resourcing and increased length of the Committee's meetings from the current length of 3 days." (AstraZeneca)

"This initiative aligns with previous proposals from industry, seeking to achieve a centralised and unified approach. To achieve these objectives, the central HTA body for all health technologies receiving Commonwealth and joint Commonwealth and State funding would need to be arm's length from government, not have a savings imperative and be empowered to engage experts relevant to the technology and submission type. This initiative has the potential to achieve improvements in time to access for codependent diagnostics and therapies. It will be important that the consistency and predictability of the current PBAC pathway in terms of reporting and timelines is maintained with the unified pathway." (Pfizer)

"In principle, (AbbVie is supportive of the streamlining and alignment of HTA pathways given the potential to reduce duplication and existing inefficiencies across current processes and pathways for all stakeholders. The triaging of submissions (2.1) will be an important Option to co-implement in order to support the expanded PBAC scope and will need to be co-designed

with Industry in order to ensure it is fit-for-purpose. It is important that this approach is adequately resourced, without impacting existing cost-recovery arrangements, and tested prior to full implementation to ensure that it achieves the desired outcome of faster, more efficient pathways. However, (AbbVie would oppose an increased remit that would transform the PBAC into a decision-making body." (AbbVie)

"Roche supports the efforts to improve the timeliness and consistency of HTA consideration for co-dependent technologies in principle. However, further detail on the expanded role of the PBAC, and the legislative amendments which will presumably underpin this expansion, will need to be understood ahead of assessing the full impact of this reform option. It is essential that the scope and breadth of the legislative changes proposed are sufficient to meet the intent of the recommendation but do not reach beyond that." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Feedback across these groups was similar, with in-principal support among many. Again, while supportive, most were keen to see appropriate checks and balances in place and for PBAC's role to remain advisory in nature.

"Supported, although PBAC will have to be differently constituted to have the expertise to address a broader range of technologies. The expanded remit of PBAC will hopefully expedite decision-making on co-dependent technologies without the need to defer until the sister committee has provided advice." (Adelaide Health Technology Assessment)

"By entrusting PBAC with a broader advisory role, stakeholders benefit from a centralised and expert evaluation process, reducing duplication and fragmentation across various HTA pathways. This consolidation promotes consistency, efficiency, and transparency in decision-making, streamlining the evaluation process for a wider range of health technologies. Furthermore, decoupling HTA recommendations through PBAC from subsequent funding decisions through the PBS enhances flexibility and responsiveness in healthcare financing." (Society of Hospital Pharmacists of Australia)

"Expanding the role (and power) of the PBAC would need to be done in a careful manner. The creation of a unified HTA pathway for all health technologies with Commonwealth funding would in theory reduce the time taken for approval of new health technologies and would certainly require legislative reform…Care needs to be taken not to shift too much power to the one body without careful checks and balances. Consultation with expert needs to be done with mutual understanding of the worth of that engagement." (Clinician - Australian Centre for Accelerating Diabetes Innovations/University of Melbourne)

"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." (Monash Children's Hospital)

Table 18. Unified HTA pathway for all health technologies with Commonwealth funding – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	16%	32%	53%	0%	19
Pharmaceutical / Medical technology company	0%	15%	15%	50%	10%	10%	20
University or research sector	0%	0%	25%	25%	50%	0%	4
Industry association / Peak body	10%	0%	10%	30%	30%	20%	10
Clinician (or representative organisation)	0%	0%	0%	60%	40%	0%	5
Consulting	33%	0%	0%	33%	33%	0%	3
State / Territory government	50%	50%	0%	0%	0%	0%	2
Other	0%	0%	25%	50%	0%	25%	4

Patients, Consumers and Representative Groups

As noted earlier, many consumer and patient advocacy groups were supportive of the move to a unified HTA pathway.

"The APAA members are supportive of a unified, national, HTA pathway for all health technology evaluation. This will enable the process to draw on appropriate specialists for all advanced therapies and technologies seeking public funding and to recommend the appropriate funding pathway." (Australian Patient Advocacy Alliance)

"Lung Foundation Australia supports this option and considers it a high priority." (Lung Foundation Australia)

"CCA support the unified, national, HTA pathway for all health technology evaluation that will draw on appropriate specialists for all advanced therapies and technologies seeking public funding and being able to recommend the appropriate funding pathway." (Crohn's & Colitis Australia)

Some stakeholders questioned whether a unified pathway would allow access to the right expertise with respect to the technology being assessed – especially for co-dependent health technologies or new and emerging health technologies.

"BCNA supports this recommendation in principle, however, has concerns about whether a unified HTA assessment pathway would ensure the correct expertise are applied to the diverse and varying range of health technologies. We are particularly interested in how codependent health technologies might be assessed by a unified HTA pathway (e.g. a new oncology drug with an associated genomic test). Where traditionally these two health technologies may be assessed separately yet co-dependently by the PBAC and the MSAC, a unified pathway might see these

assessed together for their joint therapeutic value, hopefully resulting in faster access for consumers." (Breast Cancer Network Australia)

"This has to be the goal with very clear roles and responsibilities for all stakeholders. The logistics and details require much greater development but would eliminate confusion around pathways, remove decisions about what goes where and therefore save time." (Genetic Support Network of Victoria)

"Globally, new therapies, medicines and technologies are being developed at a faster pace than ever before. This is particularly true in diabetes. The pace of change is placing a higher burden on Australia's regulatory systems. Our approvals and reimbursement framework must keep pace with these changes. There are a range of novel diabetes technologies currently available internationally that do not fit into the existing HTA policy and methods." (The Australian Diabetes Alliance),

"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." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

As per comments on the unified pathway above, most Pharmaceutical / Medical Technology Companies acknowledged this reform held potential for increasing the timeliness of access but were needing much more detail on issues such as process, resourcing and final remit. There is a strong desire for further engagement, consultation and co-design across the sector.

"This initiative aligns with previous proposals from industry, seeking to achieve a centralised and unified approach. To achieve these objectives, the central HTA body for all health technologies receiving Commonwealth and joint Commonwealth and State funding would need to be arm's length from government, not have a savings imperative and be empowered to engage experts relevant to the technology and submission type. This initiative has the potential to achieve improvements in time to access for codependent diagnostics and therapies. It will be important that the consistency and predictability of the current PBAC pathway in terms of reporting and timelines is maintained with the unified pathway." (Pfizer)

"Roche supports a unified pathway for all health technologies in principle, however, it is unclear how this reform option will specifically address simplifying and streamlining the HTA process. Roche notes this is reflected in the reform option itself which proposes investigating approaches to introduce such a pathway, and agrees that significant further detail on the framework, governance, resourcing, and committee expertise will need to be understood ahead of assessing the full impact of this reform option." (Roche Products)

"It will be imperative to have specialist input including clinical trial investigators, disease specialists as well as both regional/rural and metro perspectives. They need to be specific to the therapy being reviewed i.e. if the medicine being evaluated is for haematology, then a haematologist who specialises in that area is required, not an oncologist. Although equally knowledgeable and experts in their own right, specialists need to be specific and relevant to the area. This also is relevant to our consumer and patient organisations as well." (Antengene Australia.)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholders were also broadly supportive, albeit with similar concerns expressed regarding access to the right specialist expertise for the technology being reviewed, and the need for the new pathway to allow for the timely assessment of new and emerging technologies.

"Whilst having a unified, national, HTA pathway is a priority, SHPA continues to advocate for the development of a single-funder model for health technologies. Development of single-funder models for medicines in hospitals will reduce inequity of patient access to high-cost and complex medicines, and enable patient-centred and timely provision of treatment when and where patients require them, aligning with Australia's NMP." (Society of Hospital Pharmacists of Australia)

"The proposal if implemented will need to ensure diversity in expertise sourced for the review of speciality areas." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

"Clinicians would support the streamlining of the PBAC/MSAC into a unified HTA pathway on the proviso that appropriate funding follows this approval process. Anything that reduces the time to approval, reduces duplication and bottlenecks is welcome. Anything that slows the process down is not acceptable to clinicians or their patients." (Clinician)

"Unifying the HTA process doesn't solve the funding gap – as now with MSAC, the committee may recommend the device but have no way to fund it (see for example pressure wires in fractional flow reserve. There are also no pathways to fund digital applications prescribed by clinicians as there is now in some other developed countries)." (Medical Technology Association of Australia

Some stakeholders queried whether the intent of this reform could not be achieved by either clarifying existing pathways and processes, increasing resourcing within the current system, or both.

"The current expansion of IVD technology into genomics proteomics, biomarkers, point of care technology, and the associated digital enablers, is further evidence against the single gateway concept. It will become exceptionally challenging for a single committee, no matter how competent, to be expert enough to complete even an initial triage of potential high-medical need technology. While coordination and collaboration across HTA systems is a desirable goal, especially in so far as getting better coordinated implementation between federal and state/territory governments, we suggest this may be achieved through better resourcing processes, rather than trying to consolidate consideration of the variety of expert advisory committees into one committee and, thereby, diluting the evaluative expertise that comes from different HTA committees. We strongly suggest a single front door concept be abandoned in favour of sector specific multi-stakeholder, expert advisory groups of related healthcare professionals, service providers, patient advocacy representatives, and the industry." (Pathology Technology Australia)

2.2 Proportionate appraisal pathways

Table 19. 2.2. Proportionate appraisal pathways into account: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	71%	18%	0%	12%	17
Pharmaceutical / Medical technology company	0%	14%	59%	23%	5%	22
University or research sector	0%	33%	33%	33%	0%	3
Industry association / Peak body	0%	33%	56%	0%	11%	9
Clinician (or representative organisation)	0%	67%	33%	0%	0%	3
Consulting	0%	50%	50%	0%	0%	2
State / Territory government	0%	0%	0%	100%	0%	2
Other	0%	20%	20%	40%	20%	5

Patients, Consumers and Representative Groups

This cohort was largely supportive of the concept of proportionate appraisal pathways as a means of a more effective use HTA assessment resources. However, there were calls for strong consumer engagement and a genuine co-design of any new processes to ensure clarity and transparency of triage decision making.

"The NAA supports the proposal to develop a disease specific common model, which has the potential to benefit a number of conditions represented by the NAA." (Neurological Alliance Australia)

"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." (Rare Voices Australia)

"All appraisal pathways and triaging bodies must transparently involve consumers from the earliest stage. Consumers must be involved in creation of the PICO, including scoping and consultation." (NeuroEndocrine Cancer Australia)

"This section is unclear about the criteria that would be used for triaging which makes it difficult to comment. This would need to be totally transparent. It would also need to be monitored and reviewed to ensure that equity is being maintained." (Genetic Support Network of Victoria)

"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." (MND Australia)

Pharmaceutical / Medical Technology Companies

Pharmaceutical company stakeholders were slightly less likely to assess this reform as addressing most of the issues. Key concerns identified included the triaging process in and of itself potentially becoming a bottleneck, and any linkage made between expedited assessment and lower prices.

"Presents reasonable options for accelerating submissions such as streamlining pathways and the ability for resolution before and/or after PBAC determination, but clarity around the criteria for streamlining is required (and should not be contingent upon a price reduction). Supportive of triaging and streamlined pathways, but only if personnel have sufficient expertise to understand submission detail to ensure appropriate triaging and pathway determination." (Eli Lilly Australia)

"The options proposed cover a wide range of concepts which Roche believes will address the issue to varying extents thus conflating the response above. We note that mechanisms for early resolution of HATV and HUCN could contribute to reducing submission churn and therefore accelerate patient access. However, Roche is concerned that these meaningful gains will be overshadowed by the delays and potential failures to achieve reimbursement resulting from the pricing options proposed for cost-minimisation pathways." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

One research group noted the importance of avoiding misunderstanding at the early stages of the assessment process (including any new triaging process) through more collaborative dialogue and engagement between the applicant and the assessor. Others sought greater clarity on the scope of triaging and whether it would apply to all submission types.

"The current MSAC review process does not allow for consultation between applicant and assessor following the submission. This is an adversarial process with the "assessor" justifying their role by being as critical as possible. If an assessor makes an incorrect assumption which becomes part of the submission response it is difficult for the applicant to challenge this once presented to ESC and carried through to MSAC." (Anonymous submission).

"At present this is written as though none of this is being applied to MSAC or MedTech. Ratings given for individual proposals assume it is applied more broadly to HTA outside PBAC/PBS. However, our overall rating reflects this uncertainty." (Medical Technology Association of Australia)

Table 20. Triaging submissions – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	13%	25%	31%	31%	16
Pharmaceutical / Medical technology company	0%	5%	30%	55%	5%	5%	20
University or research sector	0%	25%	25%	25%	25%	0%	4
Industry association / Peak body	0%	0%	25%	38%	13%	25%	8
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	3
Consulting	0%	0%	50%	50%	0%	0%	2
State / Territory government	0%	50%	50%	0%	0%	0%	2
Other	0%	20%	40%	20%	0%	20%	5

Patients, Consumers and Representative Groups

As per feedback at the whole section level, most patient and consumer representative groups felt that the introduction of a triaging process would have a positive impact on their organisations and patients in terms of facilitating more timely access to health technologies.

"BCNA supports the extensive reimagining of HTA processes contained in the Options Paper, particularly the proposed new step of 'triaging' that would see new applications appropriately risk-assessed with streamlined and expedited pathways for medicines that are low-risk and target diseases with HUCN." (Breast Cancer Network Australia)

"Triaging of submissions can streamline the HTA appraisal processes by making sure that we spend the right amount of effort for each submission. However, this process should be transparent and co-designed with consumers. The framework should explicitly define HUCN, "risk and other factors", and prioritise timely and equitable access to the HTA, particularly for rare diseases." (Mito Foundation)

"CHF is not opposed to a "single front door" approach to triaging submissions, provided that such triaging ability is well resourced and does not end up becoming a bottleneck." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

Most Pharmaceutical / Medical Technology Companies provided qualified support for this reform option, noting there would need to be significant consultation and co-design with industry to ensure such a reform actually improved timeliness of assessments. There were also calls for greater initial consultation at this stage of the process (e.g. the triaging decision itself being informed through early dialogue across all key stakeholders – or at least there being an option for such dialogue if needed or requested).

"Before triaging of submissions is implemented consultation with sponsors is required to agree how far ahead of the submission triaging would be required to take place, and what information sponsors would need to provide to inform the process." (CSL Limited)

"A 'single front door approach' could work effectively. It will be important to have a simplified request process to quickly have resolution of which pathway to follow for a submission. In regard to constitution/membership for the PBAC committee and sub-committees, it will be important for industry to have visibility of who is listed and the continued pharmaceutical industry representative a part of the process." (Antengene Australia)

"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." (Alexion)

"Bayer supports in principle this option, however further clarity is required regarding; the pathway criteria, the level of information required by sponsors to facilitate triaging, and the options for sponsors to make submissions should they disagree with the determined pathway." (Bayer Pharmaceuticals ANZ)

"Roche notes that there must be clarity for sponsors around the criteria for each pathway, the information to be presented for triaging (for example, the PICO scoping step should be at the request of the sponsor to avoid inefficiencies), and the options available to sponsors if they do not agree with the decision. Roche notes that the triaging phase must be appropriately resourced so that sponsors can have meaningful interactions with departmental personnel…Roche supports the introduction of a transparent decision tree, however, the decision-making criteria, deliberations and outcomes of the triaging body must also be transparently reported." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Most of these stakeholders were supportive for this reform, albeit with a number of caveats including a strong need for transparency and clarification on exact process, and timeframes of the new process (including how this reduces assessment time vs. current practices).

"Assuming it is applied to MedTech/MSAC - this is positive and absolutely necessary if there is a move to a single HTA body in the style of NICE" (Medical Technology Association of Australia)

"Transparency to the policy and procedures for triaging will be key to ensure public, consumer and health professional confidence. Publicly noting the outcome of the triage and the next steps will also need to be made transparent." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

"Clinicians and their patients would support triaging only if it leads to improvements in timeliness of decision making and not delays. The danger lies in adding complexity and unintended consequence of subsequent delays....despite the intent of speeding access to medicines of higher clinical value. Clinicians support improving timely access to medicines for all patients.... triaging will work with the appropriate framework and enhanced transparency." (Clinician)

"I am nervous the triaging process could end up being as detailed and lengthy as the submission process and defeat the purpose. As the options paper rightly notes, the shortest possible path through the PBAC process is quite short. And this could make it even shorter and less burdensome for the sponsor and the department of health for the really simple ones. However, the triaging process does have the potential to clog up the system. Especially if the right incentives are not put in place (e.g.: taking a price cut as noted elsewhere in the options paper) means this process will be less likely to be used." (THEMA Consulting)

Table 21. Streamlined pathway for cost-minimisation submissions (therapies not claiming a significant improvement in health outcomes or reduction in toxicity) – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	14%	36%	21%	29%	14
Pharmaceutical / Medical technology company	9%	23%	23%	32%	5%	9%	22
University or research sector	0%	25%	25%	25%	25%	0%	4
Industry association / Peak body	0%	43%	14%	29%	14%	0%	7
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	2
Consulting	0%	0%	0%	50%	50%	0%	2
State / Territory government	0%	100%	0%	0%	0%	0%	1
Other	20%	20%	20%	20%	0%	20%	5

There were more divergent views expressed on this specific reform option as highlighted below.

Patients, Consumers and Representative Groups

Those groups that provided comment tended to be positive about a streamlined pathway for cost-minimisation submissions held intuitive appeal as a means of reducing time to access health technology for consumers and patients.

"APAA support streamlining a pathway for cost-minimisation submissions to avoid delay in gaining access." (Australian Patient Advocacy Alliance).

"CHF supports streamlined processes for technologies that deliver the same benefit to consumers at a cheaper price. This will also stimulate competition and lower prices. CHF understands that this will apply mostly to technologies that are not protected by intellectual property licenses." (Consumers Health Forum of Australia).

Others cautioned that there may be additional benefits to patients or consumers – beyond clinical efficacy and toxicity - that need to be factored into any price/value considerations

"We must have criteria for all therapies, being mindful they may not provide a significant improvement in health outcomes, however they may provide an improvement in quality of life and /or disease control." (NeuroEndocrine Cancer Australia)

Even though the Collaborative Consumer Group Response believed *"these options represent significant change"* and support *"transfer from specific pathways to a single entry"* they believe *"this will require ongoing consultation and communication with all key stakeholders, including consumers"*. (Collaborative Consumer Group Response)

There were concerns raised by the Collaborative Consumer Group Response though as they *"do not support options that use price as an entry point for ensuring timely access. HTA assessments should initially prioritise clinical need, safety and effectiveness separately to pricing negotiations/considerations" (*Collaborative Consumer Group Response)

Pharmaceutical / Medical Technology Companies

While most Pharmaceutical / Medical Technology Companies felt it sensible to streamline appraisal of these technologies and invest HTA resource in more complex appraisals, there was a strong rejection of the linkage between a streamlined pathway and a lower price.

"Note the positive comment is for the streamlining of the pathway only, and Janssen does not support the linking of this path to a lower price." (Johnson and Johnson Innovative Medicines)

"In principle, this can be a way to simplify and speed up access for patients. It will be important to understand the level of detail regarding the submission if the sponsor is fast-tracked straight to pricing. This pathway should not be used as an introduction to new price saving or price reduction measures. We would propose that the cost-minimisation price of the comparator is at least equal to and not less." (Antengene Australia)

"AZ believe a streamlined cost-minimisation pathway which involves abbreviated evaluation and consideration by the ESC, along with fast tracking out-of-session could speed up the HTA process. AZ agree the price of the comparator of the proposed therapy should be shared with the Sponsor early in the process prior to HTA committee consideration, however, confidential arrangements need to be sustained. AZ does not support the introduction of price reductions incentives for medicines of equivalent therapeutic value as part of the proposed cost-minimisation pathway." (AstraZeneca)

"Criteria which allow a streamlined cost-minimisation pathway would be welcomed if developed in consultation with industry. However, support for this pathway is contingent on removing the requirement to provide a lower price than the comparator. Novartis Australia cannot agree to any proposal that requires these submissions to offer or accept a lower price when claiming non-inferiority to the standard of care. Novartis supports the idea of streamlining for cost-minimisation submissions to allow for faster access for patients. However, further detail is required including: The information needed to allow a fast-track submission and at what point this is determined; What an abbreviated process would entail and how this would interact with the current meeting schedules. We note that if the submission passed through the normal process prior to consideration by ESC, the proposed process would not reduce the time to access. Hence additional detail is required before it is possible to support the option." (Novartis Australia)

"This proposal contains positive elements which propose to save resources by streamlining evaluation and fast-tracking progression to the price agreement stage. While we support such measures, maintaining price confidentiality will be a key challenge to overcome as it is likely that confidential net prices would be shared with more Sponsors in this scenario. Appropriate confidentiality must be maintained. Further consultation is required, for example, to ensure confidential information is not shared with parties who don't have a genuine interest in entering the market for a particular product." (Pfizer).

"Roche supports an abbreviated evaluation and streamlined pathway for cost-minimisation submissions in principle. However, the current approach to managing uncertainty sometimes results in sponsors either electing, or the PBAC determining, submissions follow a cost-minimisation pathway for therapies which provide a demonstrable improvement in health outcomes. Roche would be happy to supply the Reference Committee with examples on request. Roche would welcome an abbreviated evaluation if designed to reduce time to access for patients, however, this should not be at the expense of recognising the value a therapy assessed under a cost-minimisation pathway can provide. Criteria for a streamlined pathway must be developed with stakeholders and acknowledge cost-minimisation is an analytical pathway and not a reflection a therapy provides no added benefit. Many therapies also assessed under this framework provide important patient convenience benefits, clinician choice or health system efficiencies. Further consideration must also be given to the timing of the release of comparator pricing information as deliberations of the HTA committee often inform cost-minimisation calculations across therapies with different dosing regimens, treatment durations, and equi- effective doses, and the final recommended cost-minimised price. Consideration should be given to sponsors retaining the option to progress to consideration by the HTA Committee irrespective of the abbreviated evaluation out." (Roche Products)

"Alexion supports a streamlined process with many of the features outlined in the options paper. However, we have given a very negative rating because we strongly oppose options for price reductions for cost-minimised submissions, which would deny Australian patients access to many new therapies. Alexion opposes any arbitrary limitation on resubmissions. Alexion supports a streamlined pathway for comparator price sharing but only if strict Deeds of Confidentiality remain with penalties for breaches and clear timing in place of when this will be shared (to avoid price phishing) i.e. comparator has lodged submission with TGA and notice of intent for HTA submission. This should be trialled for a two-year period to ensure that this does not lead to gaming of the system and any unintended consequences." (Alexion)

"Bayer supports streamlining cost-minimization submissions however not if this requires offering a lower price or incentives offers of a lower price as set out in Option 4.1. Bayer does not support sharing of price early in process prior to HTA committee consideration as this erodes pricing confidentiality." (Bayer Pharmaceuticals ANZ)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Views on this specific reform were mixed across these stakeholder groups, with most wanting more detail on how the pathway would operate in practice, as well as concerns mandated price reductions could actually reduce consumer/patient choice and reduce timeliness of access.

"This is positive on the assumption that this is also applied to MedTech applications through MSAC. Currently it does not as written. However, the later proposal to have automatic price

reductions will likely negate all of the value of this option." (Medical Technology Association of Australia)

"May need to consider how to deal with a backlog of submissions that may be ready for this pathway once it is (if it is) implemented. It is important that this model, as all models, are fair, objective and enable opportunities for negotiation." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

"SHPA is concerned that this cost-minimisation approach will, fundamentally, reduce access to a range of medicines and limit both consumer and health professional choice of the most appropriate therapy. Australians currently have access to a range of therapies from the same class which is important as not everyone responds to every medicine in the same way. This is not something that requires reform. Australia's pharmaceutical market is less than 2% globally but remains a competitive market that should be maintained. With inappropriate measures, such as mandating cost-minimisation, sponsors may prioritise markets with more favourable pricing and reimbursement conditions. If sponsors perceive Australia's HTA process as unfavourable or uncertain, they may choose to focus their efforts on markets where they anticipate higher returns on investment. This will ultimately leave Australians with less choice of therapy." (Society of Hospital Pharmacists of Australia)

"Careful consideration should be given to how this streamlined pathway aligns with pricing policies, pricing incentives and lowest cost comparator issues. It isn't ideal that this pathway be attached to pricing discounts - especially if already being forced to cost-minimise to the lowest cost comparator. It will just encourage sponsors to seek to avoid the streamlined pathway in order to help maintain price." (THEMA Consulting)

Table 22. 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 – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	31%	23%	8%	38%	13
Pharmaceutical / Medical technology company	5%	36%	32%	18%	5%	5%	22
University or research sector	25%	0%	25%	25%	0%	25%	4
Industry association / Peak body	0%	29%	14%	29%	0%	29%	7
Clinician (or representative organisation)	0%	0%	50%	0%	50%	0%	2
Consulting	0%	0%	0%	50%	50%	0%	2
State / Territory government	100%	0%	0%	0%	0%	0%	2
Other	40%	0%	20%	0%	0%	40%	5

Patients, Consumers and Representative Groups

Most patient and consumer groups noted they did not know enough about the intricacies of the current pathway process – or the likely impact of the alternatives presented - to make an informed judgement about which reform option would best support more timely access to health technologies.

"CHF is of the opinion that the current options do not provide enough detail to ascertain which alternative will deliver the best outcome for consumers. Therefore, CHF calls for a more in-depth consultation that focuses on the four alternatives. The alternatives must be presented with case study examples, so that it will be easier to understand processes and intended outcomes. In principle, CHF is likely to support introducing an optional resolution step after HTA committee consideration, but before advice is finalised. This option ensures that consumer input is taken in consideration before the sponsor is provided information on a provisional negative recommendation by the HTA committee." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

While Pharmaceutical / Medical Technology Companies were appreciative of the intent of these reforms in terms of reducing uncertainty and potentially helping to expedite the assessment process, many had reservations on either deadlines for submissions tied to regulatory decisions in other jurisdictions, or any limitations on the number of resubmissions allowed. Price confidentiality if also considered very important, with further consideration on how such confidentiality is to be assured.

"We support all options that allow for upfront resolution of issues such as price and comparators to enable companies to determine if they should proceed with reimbursement." (UCB Australia)

"AZ believe providing earlier opportunities for engagement and a shared approach to problem solving is a major opportunity to improve HTA system efficiency outlined by stakeholders in Consultation 1. Resourcing of such an approach is critical, which includes allocation of a case manager to facilitate communication and information sharing following notice of intent to make a submission. The need for an Australian submission to be lodged within 6 months of receiving first regulatory approval from a comparable overseas regulator is overly restrictive and could limit the effectiveness of this reform. Limiting the number of submissions that could seek early resolution would also reduce the positive impact of this option, as would limiting eligibility to treatments of high added therapeutic value" (AstraZeneca)

"Supportive of sharing comparator pricing early in the process providing confidentiality maintained. The early resolution mechanism option 4 will be the most effective option, and is in line with the NICE process of commenting on the ACD. This would be appropriate for all medicines, not just those HUCN medicines that meet specific criteria. Decoupling the requirement for the TGA delegates overview can potentially have significant impact on accelerating access." (Eli Lilly Australia)

"Boehringer Ingelheim does not support an option that limits the number of resubmissions permitted." (Boehringer Ingelheim)

"Although there may be efficiency benefits in the Sponsor obtaining early ESC advice before HTA committee consideration (Alternative Option 1), MSD does not support limiting the number of resubmissions to one. There are multiple instances where ESC advice was not supported by PBAC, so conducting this process early may not resolve key economic disputes." (MSD Australia)

"Whilst aiming for the least number of submissions is ideal for all parties involved, we would still like to see the removal of only 1 submission allowed, in order to provide flexibility if required." (Antengene Australia)

"It is unclear whether an increased role of the ESC in Alternative Options 1-3 will assist the decision-making capacity for the PBAC. One of the most critical aspects of the ESC advice is whether the structure of the economic model is reliable for the PBAC's decision making. GSK has experienced situations where a model has been deemed reliable for decision making but resulted in a rejection, and the opposite situation where a model deemed unreliable has resulted in a PBAC recommendation." (GSK)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Some of these stakeholders were supportive of Option 1, albeit with similar concerns on the potential limit on resubmissions.

"Reasonable and supported, would achieve outcome of a timely and fair review process" (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

"Assuming it is applied to MedTech/MSAC - While there are many positives to this approach, the limitation of resubmission is a serious issue" (Medical Technology Association of Australia).

Others said this specific reform – including its restrictions on sponsors – risked worse outcomes than those being delivered under the current system.

One state government stakeholder wanted further clarification of how these options would impact on funding of highly specialised therapies, as well as requesting greater consultation on cost sharing arrangements – especially in terms of the current NHRA review and how specialised therapies will be funded into the future.

Table 26. 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 – impact on you

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	33%	25%	42%	12
Pharmaceutical / Medical technology company	14%	32%	27%	18%	5%	5%	22
University or research sector	50%	0%	25%	0%	0%	25%	4
Industry association / Peak body	0%	14%	14%	43%	0%	29%	7
Clinician (or representative organisation)	0%	0%	50%	50%	0%	0%	2
Consulting	0%	0%	0%	100%	0%	0%	2
State / Territory government	100%	0%	0%	0%	0%	0%	2
Other	40%	0%	20%	0%	0%	40%	5

Patients, Consumers and Representative Groups

As with the first option, some consumer and patient organisations were supportive, while others indicated they did not know how this specific reform option would impact on them and their patients.

"With regard to early resolution mechanisms for submissions of major new therapeutic advances in areas of unmet clinical need Painaustralia supports the proposal detailed in Alternative option 2: Introducing an optional resolution step before HTA committee consideration, with additional post committee resolution (2.2)." (Painaustralia)

"CCA support this as it may allow access to therapies for indications that currently do not have the high level of phase 3 registration clinical data and where these trials are very unlikely to occur due to commercial decisions e.g. fistulising disease where only luminal disease has been adequately assessed. Appropriate guidelines may encourage funding of targeted local trials to assess specific efficacy and enable funding. Data should be gathered in real world care for subsequent review and if data do not show value, access will be curtailed." (Crohn's & Colitis Australia)

Again, some consumer organisations noted the risk in limiting resubmissions and the potential negative impact his could have on sponsors bringing forward applications in a timely manner.

"This our preferred option if it allows potential areas of uncertainty to be identified early and provides iterative opportunities to discuss, review and seek consumer/clinician evidence to address these uncertainties without the need for multiple resubmissions. The current mechanism of resubmissions to address uncertainties represents a delay in access for patients. We do have a concern in the option about the proposal to limit options for sponsors to resubmit in the case of a negative recommendation. This could have unintended effect of certain technologies being

exited from HTA processes with no pathways available for patients to access them in the future. All options should provide maximum opportunity for uncertainties to be resolved in the initial process." (Rare Voices Australia)

"Prescribing a limited number of resubmissions – we support all efforts to avoid multiple resubmissions as resubmissions represents a delay in access however mandating a limited number of resubmissions may have the unintended effect of disincentivising submissions where this is a high level of uncertainty." (Collaborative Consumer Group Response)

Pharmaceutical / Medical Technology Companies

A number of Pharmaceutical / Medical Technology Companies were unsupportive of Option 2, with many questioning how this differed to current HTA practice.

"No obvious benefit over Option 1, given we already have this process in place." (Eli Lilly Australia)

"It is unclear whether an increased role of the ESC in Alternative Options 1-3 will assist the decision-making capacity for the PBAC. One of the most critical aspects of the ESC advice is whether the structure of the economic model is reliable for the PBAC's decision making. GSK has experienced situations where a model has been deemed reliable for decision making but resulted in a rejection, and the opposite situation where a model deemed unreliable has resulted in a PBAC recommendation." (GSK)

"Not supported, too early in the process" (Biogen)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

One stakeholder indicated that if taken forward, this option would need more definitive feedback from ESC to ensure the changes made before resubmission are addressing the specific concerns or deficiencies identified.

"ESC is not currently a decision-making committee. Currently most of the ESC advice is based on the Commentary's Executive Summary and main issues raised. To undergo a resolution step there needs to be clear direction regarding what submission elements are/are not acceptable to the committee and what points raised in the evaluation are valid and need addressing by the sponsor. Without a clear direction/signal from a decision-making committee it is unlikely that the resolution process will proceed satisfactorily. We canvassed a post-ESC/pre-PBAC resolution step in Paper 1 but this was not preferred over a post-PBAC resolution step, partly because PBAC makes a decision while ESC does not. If, however, the nature of ESC changes and it becomes more definitive about the deficiencies in the submission that need amending, then this approach might work. However, I disagree with the additional post committee resolution because then the process of "resubmission churn" would just be similar to the current process - and the point of the Review was to reduce resubmission churn and speed up patient access to medicines. If there is no possibility of resubmission and the resolution timeframe is mandated, then both parties (Government/Government-related agents and industry) would have greater incentive to reach a solution." (Adelaide Health Technology Assessment)

Table 27. Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN: Alternative option 3: Early Price negotiation – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	8%	17%	8%	8%	8%	50%	12
Pharmaceutical / Medical technology company	14%	27%	32%	14%	5%	9%	22
University or research sector	25%	0%	50%	0%	0%	25%	4
Industry association / Peak body	0%	14%	14%	29%	14%	29%	7
Clinician (or representative organisation)	0%	0%	50%	50%	0%	0%	2
Consulting	0%	0%	0%	100%	0%	0%	2
State / Territory government	100%	0%	0%	0%	0%	0%	2
Other	40%	0%	20%	0%	0%	40%	5

Patients, Consumers and Representative Groups

While some consumer groups noted their frustration with the current system - resultant positive recommendations but informal price negotiations delaying access - others were concerned that this option elevated price above the more important considerations of safety, efficacy and need.

"CCA supports earlier price negotiation in the process - the current situation of positive PBAC recommendation to then have a non-sustainable cost offered is disheartening for patients and clinicians and a huge waste of resources." (Crohn's & Colitis Australia)

"We do not support this option as we believe that price negotiation should not be initial framing factor for HTA decisions - these should based on clinical effectiveness and unmet need. We would be concerned that leading with would have unintended flow on implications including stalling assessments for HUCN/HATV therapies." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

Some companies were supportive of this option given its scope to prevent wasted effort and resources but price is likely to be a key challenge. Others felt this was not different from the current system that allows for price to be changed pre-PBAC response.

"We support all options that allow for upfront resolution of issues such as price and comparators to enable companies to determine if they should proceed with reimbursement." (UCB Australia)

"We are supportive of early price negotiation in the process before recommendation, if this facilitates faster implementation in a defined time period once the submission is recommended," (Antengene Australia)

"No significantly different from current system with ability to change price in pre-PBAC response." (Eli Lilly Australia)

"Not supported, too early in the process" (Biogen)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Some stakeholders suggested this option held potentially the greatest scope to reduce timelines, but that further detail was needed.

"This option may bring about the most improvement in timelines. Transparency, a values framework, and early adoption of price negotiation may reduce barriers and improve timelines. If the horizon scanning model is adopted then early price negotiation will be a must have to the success of considering best available HTA." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

"As mentioned above, if the nature of ESC changes and it becomes more definitive about the deficiencies in the submission that need amending and signals the price that is likely to be acceptably cost-effective, then this early price negotiation approach might work." (Adelaide Health Technology Assessment)

One state government stakeholder mentioned that for products that will be funded through the NHRA and where States and Territories contribute 50% of the cost, the States and Territories should be consulted during any price negotiation.

Table 28. 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 – impact on you/organisation

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	8%	25%	17%	8%	0%	42%	12
Pharmaceutical / Medical technology company	0%	18%	18%	55%	5%	5%	22
University or research sector	25%	0%	25%	0%	25%	25%	4
Industry association / Peak body	0%	0%	29%	43%	0%	29%	7
Clinician (or representative organisation)	0%	50%	50%	0%	0%	0%	2
Consulting	0%	0%	50%	50%	0%	0%	2
State / Territory government	100%	0%	0%	0%	0%	0%	2
Other	40%	0%	20%	20%	0%	20%	5

Patients, Consumers and Representative Groups

Some consumer groups noted this option could help in reducing delays to access, but again noted concerns with capping the number of resubmissions possible.

"This option combined with Option 2 could enable committees to check broader implications of recommendations with all stakeholders to ensure recommendation meets needs of clinicians and consumers…We do have a concern in the option about the proposal to limit options for sponsors to resubmit in the case of a negative recommendation. This could have unintended effect of certain technologies being exited from HTA processes with no pathways available for patients to access them in the future. All options should provide maximum opportunity for uncertainties to be resolved in the initial process." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

Pharmaceutical companies commonly felt this option was the most preferred given the timing for re-engagement – but many qualified their support and indicated a need for greater detail.

"This would be the appropriate time for early resolution process to start." (Biogen)

"The proposed early resolution process (Alternative Option 4) should occur after HTA committee consideration but before advice is finalised. Current early re-entry pathways are working well and must be retained alongside any new early resolution mechanisms for technologies that address areas of HUCN. MSD is supportive of early resolution pathways for HUCN after HTA committee consideration but before advice is finalised as per Alternative Option 4. Different perspectives between MSD and the PBAC on clinical and economic uncertainties have been the key driver of submission churn." (MSD Australia)

"Alexion supports option 4 as the best way of providing more effective and streamlined HTA processes. The requirement for submissions to be lodged within six months of first international registration is unrealistic and would severely curtail the effectiveness of the proposed scheme and the ability of sponsors to participate. Any requirement to create a nexus between international registration and Australian listing timeframes should be removed to ensure sponsors can more confidently enter the HTA appraisal process ensuring all requirements of a successful submission can be made. Arbitrary time requirements such as that proposed could mean sponsors may not be able to lodge a submission in Australia given international considerations." (Alexion)

"Preferred option, but provisional negative recommendation must be versus most recent communication with sponsor (i.e. pre-PBAC advice). Should not be "recommendation" and be dramatically different to sponsor proposal. Option to provide additional supportive evidence relevant to price negotiation. RSA negotiations should also be considered at this time." (Eli Lilly Australia)

"Bayer is in principle supportive of introducing an optional resolution step after HTA committee consideration but before advice is finalised. We agree with other stakeholders that this approach requires further co-design and reconsideration of the proposed criteria for this option. The criteria to insist submissions are made to the PBAC and TGA at the same time is overly restrictive and fails to recognize that it is not always possible to submit to TGA and PBAC in parallel; the choice

and speed of regulatory pathway is an important consideration and not necessarily under the decision-making authority of the Australian subsidiary of a sponsor company. While it is recognised multiple resubmissions are burden to all parties, Bayer suggests capping the maximum allowable number of submissions risks reducing patients access." (Bayer Pharmaceuticals ANZ)

"Roche supports the introduction of early resolution mechanisms for cost-effectiveness submissions, however clear criteria to determine what are considered major new therapeutic advances, and deemed areas of HUCN, are needed. Consideration should also be given to the need to satisfy a submission timeframe of 6 months from the first regulatory approval in comparable international jurisdictions. There can be several factors as to why it would not be practical for a submission to be made within that time frame, and should therefore not be a condition of eligibility for early resolution mechanisms. For example, there is a substantial difference in the suitable level of evidence to support early phase clinical data for a regulatory application versus that needed to support a reimbursement decision, which could result in timeframes of more than 6 months between regulatory approval and the preparation of a reimbursement submission. Roche also does not support any cap on the allowable number of resubmissions as this unfairly denies patients access. Roche supports the introduction of early resolution mechanisms in general, however, this is the preferred option. To effectively use an early resolution mechanism and ultimately avoid negative recommendations and resubmission churn, it is important that all evaluation considerations and positions, including those of the HTA Committee, are available to inform the resolution process itself." (Roche Products)

"As currently presented only Alternative Option 4 presents a viable way forward as it retains the importance of the PBAC in determining the value of the intervention in question. However, until further detail is provided it is not possible to support any option that appears to amend the current streamlined pathways." (Novartis Australia)

"In its current form, this seems to be a 'resubmission' without it being called a resubmission because it still can take one full cycle (i.e. 17 weeks) to resolve. This is not supporting faster access to medicines." (Antengene Australia)

"While Option 4 appears the most appropriate, among the options proposed based on the available information, we are concerned about the application of specific time pressure on negotiations. It is in the interest of both the government and sponsors to come to early agreement. In the status quo Australia's value framework can be an obstacle to reaching agreement and so any improvements to these pathways needs to be coupled with an improved recognition of value of innovative medicines. It is also important the options for further resubmissions are not curtailed by failure to reach an outcome via this pathway. Bridging funding that provides early access is important in addressing patient need as quickly as possible with inclusion of an arrangement for review and exit if required." (Pfizer)

Table 29. Expanding resolution step to all relevant cost effectiveness submissions – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	8%	50%	8%	33%	12
Pharmaceutical / Medical technology company	0%	10%	24%	48%	14%	5%	21
University or research sector	33%	0%	33%	0%	33%	0%	3
Industry association / Peak body	0%	0%	17%	33%	17%	33%	6
Clinician (or representative organisation)	0%	0%	100%	0%	0%	0%	2
Consulting	0%	0%	50%	50%	0%	0%	2
State / Territory government	100%	0%	0%	0%	0%	0%	1
Other	50%	0%	25%	0%	0%	25%	4

Patients, Consumers and Representative Groups

No notable comments were received on this specific reform option among consumer and patient organisations.

Pharmaceutical / Medical Technology Companies

Several Pharmaceutical / Medical Technology Companies expressed a degree of support for this specific reform option, but again with several points of qualification of this support.

"This is highest priority - ensuring ALL medicines (and patients) benefit from process improvements and earlier access. These will generally be offering significant improvements to new patient groups." (Eli Lilly Australia)

"BMSA believes that the options outlined in Chapter 2.2 specific to early resolution need to be expanded to all cost-effectiveness submissions in order to improve timely access to medicines for Australian patients. Gaining as much alignment as possible on the decision problem and analytical approach at an early stage of the HTA process will be more efficient than addressing these issues via re-submissions. BMSA believes that a target of 100 days from TGA registration to PBS listing should be the aim for applications where clinical outcomes are improved (i.e. cost- effectiveness submission) – and that a system set up for early dialogue and agreement across all stakeholders on value and sharing of uncertainty would deliver on such an aim. Improvements with regards to transparency and timelines within the post PBAC process would also assist with delivering on a target of 100 days from TGA registration to PBS listing." (Bristol Myers Squibb Australia)

"Alexion supports the expansion of the early resolution step to all submissions at the earliest opportunity." (Alexion)

"Given the uncertainty with what therapies qualify as HATV in areas of HUCN, Roche supports expanding the resolution step to all cost-effectiveness submissions after a pilot as soon as practicable. We note that a pilot covering one to two PBAC cycles would be appropriate; expanding the resolution step would drive faster access for patients. Subsequently, the pilot should also extend to include non-HATV therapies, given the uncertainty around the definition of HATV." (Roche Products)

"The proposal aims to extend the early resolution step to all relevant cost-effective submissions following a pilot. However, it is crucial that the pilot occurs as promptly as possible, ideally within one or two PBAC cycles, to expedite access for patients without unnecessary delays." (Boehringer Ingelheim)

"This should be implemented as soon as possible after the pilot in medicines for area of HUCN. It will be important not to lose the benefit the current resubmission pathways (such as early re- entry). These must not be disbanded without industry agreement." (A.Menarini Australia)

"Since the proposal is for expansion only following a pilot phase, it will be important to complete any pilot quickly to achieve the goal of faster access for all health technologies which, by seeking listing on cost-effectiveness basis, are anticipated to provide health benefits to patients. We also reiterate concerns raised regarding the mechanisms raised for the pilot phase apply for this option as well." (Pfizer)

Table 30. Development of a disease specific common model (reference case) for disease areas with high active product development – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	55%	27%	18%	11
Pharmaceutical / Medical technology company	27%	36%	23%	9%	0%	5%	22
University or research sector	0%	0%	50%	0%	25%	25%	4
Industry association / Peak body	17%	17%	33%	17%	0%	17%	6
Clinician (or representative organisation)	0%	50%	0%	0%	50%	0%	2
Consulting	0%	50%	0%	50%	0%	0%	2
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	50%	0%	50%	0%	0%	0%	4

There was a diversity of opinion on this proposed reform as highlighted in the issues raised below.

Patients, Consumers and Representative Groups

Patient and consumer groups were broadly supportive of this reform option, albeit with a proviso that any common disease models would need to be developed in close consultation with those who have expert knowledge of the specific disease or condition. There was also a concern raised around the complexities of new technologies that may impact across more than one condition/disease.

"This would seem to be a really productive use of resources and benefit sharing. It would be important to review for equity over time." (Genetic Support Network of Victoria)

"These need to be generated with strong consumer engagement." (MND Australia)

"Painaustralia supports the development and adoption of a consistent model structure for specified disease areas where there are several potential therapies/ technologies under development (as identified through horizon scanning). Painaustralia emphasises that this will require input from a wide range of stakeholders to ensure a comprehensive representation of the disease area. In Painaustralia's view the development of disease specific models would strengthen and support consistency in decision-making as models across different technologies for the same disease/condition will be more easily comparable." (Painaustralia)

"There is some concern that this risks examination of the use of medications for alternative disease groups, thereby slowing access to medications across a wider disease profile." (Crohn's & Colitis Australia)

Pharmaceutical / Medical Technology Companies

There was generally less support for this option among Pharmaceutical / Medical Technology Companies. Many suggested developing such models initially would be time and resource intensive, and there would also need to be ongoing investment to keep such models current and relevant.

"Considered inefficient use of resourcing given other options in paper." (Eli Lilly Australia)

"The complexity of and resourcing required for the development and maintenance of a common model would outweigh the usefulness of this approach in reducing time to access. This model has been tested in overseas jurisdictions with little success. Australia does not have capability or capacity to lead in this area." (A.Menarini Australia)

"Bayer does not support this option and would agree with other stakeholders that is not required and will not deliver on reducing time to access for Australian patients or ensure our assessment processes keep pace with rapid advances in health technology. Seeking input from stakeholders to ensure the model is comprehensive in its representation of the disease area will require resources that could be better directed to other options designed to speed access. Where disease specific models have been developed in other jurisdictions, it has been difficult to create a model that captured sufficiently the complexity to enable use by multiple sponsors; this is especially problematic where parameters are different in relation to patient population, lines of therapy or disease stage. To accommodate parameter differences can require simplification assumptions that adds to parameter uncertainty and risks the full value of the therapeutic benefit

not being captured. Where there is continued changes in disease management and/or standard of care; disease-specific common models will, without updates, become redundant." (Bayer)

"As demonstrated by NICE's adoption of a common model for technology appraisal of Covid-19 medicines, the development of disease-specific common models is a complex endeavour and challenging to implement. Ongoing revision of these models is critical as standards of care evolve and the understanding of long-term disease outcomes changes based on emerging evidence: they must be dynamic and are therefore highly resource intensive. Common economic models require a degree of flexibility in order to allow Sponsors to account for unique clinical features (treatment administration, benefit and risk profile) and demonstrate the value of a specific product. A non-specific model could simply be a "blunt economic tool" that doesn't effectively recognise or value innovation of either a major or incremental nature. The development of disease specific common models should be considered in the broader context of other Options intended to support a proportionate approach to submission appraisal. (AbbVie considers that given the resourcing and capacity challenges within the current system, time and effort would be more appropriately allocated to better support the implementation of other Options that will have a greater impact on improving timely access to medicines for patients." (AbbVie)

"Roche does not support the introduction of a disease specific common model. Roche notes that sequential listing of multiple therapies on the PBS targeting the same population, largely means that therapies listed on the basis of cost-minimisation are "accepting" the parameters that determined cost-effectiveness for the first therapy in the first instance, and it is unclear where the efficiencies with this option lie. Further, Roche has noted that the experience with disease-specific models for non-small lung cancer (NSCLC) has been trialled in the UK. Based on Roche UK's experience during this trial, it has been difficult to create a model that captures sufficient complexity to enable it to be used by multiple sponsors where parameters have been flexible enough to accommodate different patient populations (and subpopulations) drug classes, disease stages, lines of therapy (and impact on subsequent lines of therapy), dosing regimens and all components of an economic model. Failure to reflect this complexity in these models will likely lead to inaccuracies in capturing the full value of the therapy. Rapid changes in disease management and standard of care will also lead to the rapid redundancy of these models. It is noted that the effort required to develop a range of workable disease-specific common models would likely outweigh any efficiencies gained, and those resources would be better directed to other options where there is more certainty of accelerating access." (Roche Products)

"Development of a disease specific common model is likely to have an unintended consequence of delaying time from registration to reimbursement by introducing greater complexity and therefore greater uncertainty into HTA decision-making. Enhancing the PICO scoping process would help address modelling uncertainties and define key assumptions to improve HTA efficiency" (AstraZeneca)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

While these models hold a degree of intuitive appeal for some stakeholders, others noted a range of practical challenges and also the perceived limited success in the development of such models in other jurisdictions.

"Whilst this sounds like a well-meaning reform, the unintended consequence of over- simplification may cloud the complexities of the care pathways... To me, this sounds difficult to

realise. Clinicians can continue to provide ongoing, specific, real time advice in a timely manner if approached in good faith with respect and transparency." (Clinician)

"Probably no real gain for industry-sponsored submissions. NICE has not made any significant progress with this concept since it was announced in the UK" (Medical Technology Association of Australia)

Table 31. Decouple the requirement for the TGA Delegate's overview to support PBAC advice – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	8%	25%	25%	42%	12
Pharmaceutical / Medical technology company	0%	9%	17%	48%	26%	0%	23
University or research sector	0%	25%	25%	25%	0%	25%	4
Industry association / Peak body	0%	0%	33%	50%	0%	17%	6
Clinician (or representative organisation)	0%	0%	0%	33%	67%	0%	3
Consulting	0%	0%	50%	50%	0%	0%	2
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	25%	25%	25%	0%	0%	25%	4

Patients, Consumers and Representative Groups

Some consumer and patient groups were positive towards this specific reform option as a means to improve timeliness of access.

"Parallel processing holds a lot of promise. The current two-stage process continues to add unnecessary delays to access in Australia." (Mito Foundation)

"We must ensure that it is a full parallel processing." (NeuroEndocrine Cancer Australia)

"We support this as the PBAC should be given authority and capacity to make recommendations in line with agreed guidelines and not limited by other processes/systems. This option will help to ensure the PBAC decisions can be timely, equitable and responsive to complexity and nuance in rare disease technologies." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

Several Pharmaceutical / Medical Technology Companies provided in-principal support for this reform, albeit with a number of qualifications and issues requiring consideration if taken to implementation.

"Novartis would support this decoupling if there were not an implied requirement for early PBAC submission and that it was at the discretion of the sponsor when the product was submitted to the PBAC." (Novartis Australia)

"This proposal will permit increased use of parallel processing which will assist in achieving faster patient access. To be successful, there must be a mechanism by which potential differences in indications recommended by the delegate and included in the PBAC submission can be quickly resolved." (Pfizer)

"Bayer supports this option has the capacity to reducing time to access for Australian patients assuming in situations where PBAC would be minded to recommend, that this allows for post PBAC recommendation processes to be commenced while awaiting either the delegate's overview or ARTG listing." (Bayer Pharmaceuticals ANZ)

"Roche supports decoupling the requirement for the TGA Delegate's overview to support PBAC advice, however, without further detail on how this will be managed (e.g. will other post-PBAC processes also continue in the absence of the TGA Delegate's overview) it is unclear how this reform option will reduce time to access for patients." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was some support for this reform option across these stakeholders, albeit with some concern regarding resourcing and whether a parallel process may pose challenges for evaluation teams and committees.

"Enabling the PBAC to communicate its likely advice to sponsors before receiving the TGA delegate's overview, promotes efficiency and transparency in the funding and assessment pathways." (Society of Hospital Pharmacists of Australia)

Table 32. Case manager – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	8%	31%	38%	23%	13
Pharmaceutical / Medical technology company	5%	5%	27%	41%	14%	9%	22
University or research sector	0%	0%	25%	25%	25%	25%	4
Industry association / Peak body	0%	0%	33%	33%	17%	17%	6
Clinician (or representative organisation)	0%	0%	33%	0%	67%	0%	3
Consulting	0%	0%	50%	50%	0%	0%	2
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	25%	0%	50%	0%	25%	0%	4

The proposed introduction of greater case management capacity for submissions was strongly supported across all stakeholder groups.

Patients, Consumers and Representative Groups

Consumer groups supported this reform option, albeit with many noting those performing this role need the appropriate skills and knowledge to provide value and ideally helping to proactively drive issues to timely resolution.

"There would be real value in formalising this role and making it transparent to all stakeholders." (Genetic Support Network of Victoria)

"Depends on the definition of their role and their expertise. Could potentially be positive but could also reduce efficiency." (Mito Foundation)

"It is essential the Case Manager is familiar and knowledgeable of the needs of the specialty health condition and the impact on those patients." (NeuroEndocrine Cancer Australia)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies were broadly supportive of the case manager reform option, albeit with greater clarification needed on the specific role and remit of this new function.

"Earlier and more frequent interactions with evaluators are much needed but the current case management role in Pricing Pathway A will need to be improved to achieve this. MSD welcomes the opportunity for more interactions with evaluators. Currently the limited opportunity for Sponsors to address questions during the commentary response process can result in issues that have not been clarified by the time of PBAC consideration. This is particularly true for new disease areas, where treatment pathways and clinical considerations are not well understood. The lack of early and ongoing interactions between evaluators, committee members, Sponsors and other stakeholders contributes to submission churn and delays in access. This issue was highlighted by the Reference Committee, who noted that Sponsors frequently rely on the PBAC decision from their first submission as a form of early advice to inform the development of a more fulsome latter submission. MSD also support resourcing a case manager to facilitate communication between MSD and the Department regarding key economic issues." (MSD Australia)

"A case manager (and a backup) assigned to each CUA/CEA submission is welcomed. It will be important to understand what their remit is in terms of communication and information sharing, as if we can have discussions in real-time then we may be able to resolve any questions or issues in a timely manner." (Antengene Australia)

"In principle, Boehringer Ingelheim supports the proposal to assign a case manager for each cost-effectiveness submission. However, further detail regarding the scope of the case managers responsibilities and their interactions with the sponsor is required to be able to assess the usefulness and any unintended consequences." (Boehringer Ingelheim)

"Bayer supports the concept of a case manager; however, the remit of this role should be co-designed with industry and other sponsors to ensure this role will add value. It will be important to take learnings from the use of the current case management approach used to progress pricing pathway A in order to enhance a case manager role for submissions. It is unclear whether this case manager would act as an end-to-end facilitator, most value would come from a single person facilitating communication and information sharing prior to submission and also to assist in progressing pricing." (Bayer Pharmaceuticals ANZ)

Some queried the additional costs this new role would add and whether this would be justified.

"Considered inefficient use of resourcing given other options in paper." (Eli Lilly Australia)

"We are concerned this proposal won't lead to faster outcomes and may create new costs. Our experience with Pricing Pathway A, the model for this proposal, is that it doesn't improve efficiency or timeliness of the process and therefore is of limited utility despite the significantly higher fees. Feedback should be sought from participants in the Pathway A model to ensure efficiency benefits of case manager are realised." (Pfizer)

Comparing the 2.2 Alternative options

Table 33. Introducing an optional resolution step before HTA committee consideration: How well alternative option addresses issues by stakeholder type

	To a significant extent	To a moderate extent	To a limited extent	Not at all	Don't know	Sample size
Patient or consumer (or representative organisation)	20%	40%	20%	0%	20%	10
Pharmaceutical / Medical technology company	0%	11%	61%	22%	6%	18
University or research sector	0%	0%	33%	0%	67%	3
Industry association / Peak body	0%	33%	17%	17%	33%	6
Clinician (or representative organisation)	0%	50%	0%	0%	50%	2
Consulting	0%	100%	0%	0%	0%	2
State / Territory government	0%	0%	0%	100%	0%	2
Other	0%	25%	0%	25%	50%	4

Table 34. Introducing an optional resolution step before HTA committee consideration, with additional post committee resolution: How well alternative option addresses issues by stakeholder type

	To a significant extent	To a moderate extent	To a limited extent	Not at all	Don't know	Sample size
Patient or consumer (or representative organisation)	30%	30%	20%	0%	20%	10
Pharmaceutical / Medical technology company	6%	11%	56%	22%	6%	18
University or research sector	0%	0%	0%	33%	67%	3
Industry association / Peak body	0%	50%	0%	17%	33%	6
Clinician (or representative organisation)	0%	0%	50%	0%	50%	2
Consulting	0%	100%	0%	0%	0%	1
State / Territory government	0%	0%	0%	100%	0%	2
Other	25%	25%	0%	0%	50%	4

Table 35. Early price negotiation: How well alternative option addresses issues by stakeholder type

	To a significant extent	To a moderate extent	To a limited extent	Not at all	Don't know	Sample size
Patient or consumer (or representative organisation)	20%	10%	20%	10%	40%	10
Pharmaceutical / Medical technology company	11%	6%	33%	39%	11%	18
University or research sector	0%	33%	0%	0%	67%	3
Industry association / Peak body	17%	33%	0%	17%	33%	6
Clinician (or representative organisation)	50%	0%	0%	0%	50%	2
Consulting	50%	50%	0%	0%	0%	2
State / Territory government	0%	0%	0%	100%	0%	2
Other	0%	25%	0%	0%	75%	4

Table 36. Introducing an optional resolution step after HTA committee consideration but before advice is finalised: How well alternative option addresses issues by stakeholder type

	To a significant extent	To a moderate extent	To a limited extent	Not at all	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	20%	40%	20%	20%	10
Pharmaceutical / Medical technology company	32%	32%	26%	5%	5%	19
University or research sector	33%	0%	0%	0%	67%	3
Industry association / Peak body	0%	50%	17%	0%	33%	6
Clinician (or representative organisation)	0%	0%	50%	50%	0%	2
Consulting	50%	50%	0%	0%	0%	2
State / Territory government	0%	0%	0%	100%	0%	2
Other	25%	25%	0%	0%	50%	4

Patients, Consumers and Representative Groups

Patients, Consumers and Representative Groups found it difficult to determine the best of the alternative options as also seen above and in earlier comments.

"Assessing the potential impact of these alternate options are outside the scope of Mito Foundation's expertise. However, we would like to stress that the option chosen should ensure transparency (including defining HUCN), and timely and equitable access to therapeutics for all Australians, including those with rare/ultra-rare diseases." (Mito Foundation)

"We have selected Option 2 as the Option most likely to address issues, however any option that creates provision for submissions associated with high levels of uncertainty that will reduce the need for reduce submission and improve time to access should be considered. We support all options to reduce resubmissions but do not support aspects that limit the number of resubmissions as this could make HUCN/HATV therapies permanently unavailable to patients - alternatives need to be considered." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

On balance, most Pharmaceutical / Medical Technology Companies tended to favour Option 4 – albeit with several qualifications noted.

"Option 4 is the strongest as it occurs post HTA Committee assessment and involves the decision makers in the process unlike other options." (Johnson and Johnson Innovative Medicines)

"The proposed early resolution process (Alternative Option 4) should occur after HTA committee consideration but before advice is finalised. Current early re-entry pathways are working well and

must be retained alongside any new early resolution mechanisms for technologies that address areas of HUCN." (MSD Australia)

"Based on Alexion's engagement with the HTA assessment process, option 4 is most likely to lead to more timely decision-making and reduce resubmission churn. However, Alexion does not support restricting resubmissions which could diminish the positive impacts of this pathway and would potentially deny Australian patients access to new therapies." (Alexion)

"The early resolution mechanisms proposed may not be the best approach for achieving patient access in areas of HUCN because it's unclear any of these would be substantially faster and as there is no bridging funding available to ensure early access to these therapies is not undermined by pricing considerations that may require some time to resolve. While Option 4 appears the most appropriate, among the options proposed based on the available information, we are concerned about the application of specific time pressure on negotiations. It is in the interest of both the government and sponsors to come to early agreement. In the status quo Australia's value framework can be an obstacle to reaching agreement and so any improvements to these pathways needs to be coupled with an improved recognition of value of innovative medicines. It is also important the options for further resubmissions are not curtailed by failure to reach an outcome via this pathway. Bridging funding that provides early access is important in addressing patient need as quickly as possible with inclusion of an arrangement for review and exit if required." (Pfizer)

"Alternative option 4 would need further consideration but offers best opportunity to maintain speed to access. It has similarities to the current early re-entry pathway, though offers the opportunity to understand resolve and issues that the HTA committee has raised without awaiting PBAC minutes. Introducing resolution step before HTA committee consideration would add to HTA process for little gain, especially if the HTA Committee were not supportive of the changes made to a submission agreed within resolution process." (Bayer Pharmaceuticals ANZ)

Table 37. Reform option you think offers greatest scope to improve the HTA assessment process by stakeholder type

	Alternative option 1: Introducing an optional resolution step before HTA committee consideration	Alternative option 2: Introducing an optional resolution step before HTA committee consideration, with additional post committee resolution	Alternative option 3: Early Price negotiation	Alternative option 4: Introducing an optional resolution step after HTA committee consideration but before advice is finalised	None of these	Sample size
Patient or consumer (or representative organisation)	17%	42%	25%	17%	0%	12
Pharmaceutical / Medical technology company	0%	5%	11%	79%	5%	19
University or research sector	0%	0%	0%	67%	33%	3

Industry association / Peak body	0%	0%	67%	33%	0%	6
Clinician (or representative organisation)	0%	0%	50%	0%	50%	2
Consulting	0%	50%	50%	0%	0%	2
State / Territory government	0%	0%	0%	0%	100%	2
Other	0%	0%	40%	20%	40%	5

Section 3: Methods for HTA for Australian government subsidy (technical methods)

Stakeholders were invited to provide written comment on the reform options presented for methods for Australian government subsidy (technical methods) as per the table below (reproduced from the HTA Review's Options Paper).

Subject	Key option/s
3. Methods for HTA for Australian Government Subsidy (technical methods)	
3.1. Determination of the Population, Intervention, Comparator, Outcome (comparator is also addressed under economic evaluation)	
Increased early stakeholder input	Increased early input on the PICO from patient and clinician communities to ensure all relevant patient populations that could potentially benefit from the new therapy are considered in the HTA, and to identify issues that may impact implementation early to be addressed (for new drugs or major expanded indications claiming added therapeutic value).
Increased transparency for stakeholders	That plain language summaries of the PICO are produced in collaboration between the sponsor and the Department to be released with the PBAC agenda to increase transparency about the proposed treatment population and communicate the expected benefit (outcome) to assist in managing stakeholder expectations (for new drugs or major expanded indications claiming added therapeutic value).
Updated guidance	Updated guidance to require the explicit consideration of health equity and priority populations for new treatments. Additional guidance be produced regarding when and how PICO is to be developed, to ensure criteria of importance to patients and clinicians (e.g. for HATV/HUCN reasons) are appropriately considered and discussed.
3.2. Clinical Evaluation Methods	
Overarching principles for adopting methods in Australian HTA	Implement the overarching principles for adopting methods in Australian HTA as outlined in the HTA Review Paper on Clinical Evaluation Methods in HTA for all HTA Methods.

Methods for the assessment of nonrandomised and observational evidence	Update methods relating to the assessment of nonrandomised and observational evidence as outlined in the HTA Review Paper on Clinical Evaluation Methods in HTA in line with the overarching principles mentioned above. 1. Methods relating to indirect comparisons: a. Require the presentation of a comparison of study characteristics, as well as how successful efforts for controlling for differences in characteristics are likely to be. 2. Methods relating to the creation of control groups: b. Require justification of why an indirect comparison is not possible, or less reliable, than the proposed approach of creating a control group. c. Require justification for the use of methods that are not prespecified in the study protocol of the proposed technology. d. Require multiple approaches and/or multiple data sources, if possible, and a discussion of any inconsistencies in estimates. 3. Methods relating to the use of nonrandomised studies - the use of nonrandomised studies to estimate a treatment effect should be: a. well justified, b. prospectively designed (preferably in collaboration with HTA or regulatory scientific advice) c. registered, and d. supported by multiple sensitivity analyses and transparently reported. 4. Methods relating to adjustment of the treatment effect in the presence of treatment switching a. Require multiple methods to be reported to show consistency of the results. This may include alternative approaches (not only methods to adjust for treatment switching) such as translating intermediate endpoints unaffected by treatment switching into final outcomes. b. Require a justification of the use of methods that are not pre-specified in the trial protocol of the key study for the proposed technology. 5. Methods relating to the use of RWD and RWE in HTA: a. Greater guidance for the use of RWD and RWE in HTA is required. As well as a curated list of methods that may be used to generate RWE, guidance should consider what data sources would be acceptable for particular purposes (e.g. costs, utilities, treatment effect). Guidance should also adopt a terminology that defines different sources of RWD more precisely than the umbrella term of "RWD". b. Specific guidance is required regarding the assessment of the quality of the data source, and it may be an option to require a minimum standard of data quality prior to use in HTA. c. RWE should not be acceptable to use for the purpose of determining treatment effectiveness of a technology unless the following conditions are met, or there is a strong justification that they cannot be met: i. the technology is for use in a population with a HUCN ii. higher quality evidence cannot be generated, or will not be generated in a timely fashion iii. multiple sources of RWE are presented (including both methods of generating RWE from a source, and multiple RWD sources), and

	iv. the use of RWE is prespecified in the study protocol for the proposed technology
Methods for the assessment of surrogate endpoints	Implement the options relating to the methods relating to the use of surrogate endpoints as outlined in the HTA Review Paper on Clinical Evaluation Methods in HTA in line with the overarching principles mentioned above. Namely: 1. Guidance for the use of surrogate endpoints in HTA should include circumstances where surrogates would be acceptable (and may include a list of previously accepted surrogate endpoints paired with use cases). Guidance should also revisit methods required to validate surrogates to ensure they are achievable by industry and include methods for describing the uncertainty in the use of surrogate endpoints, particularly where surrogate relationships are used in combination with other methods (such as indirect comparisons or model extrapolation) where uncertainty may be substantially increased. 2. Guidance for the evaluation of evidence using surrogate endpoints is required and should include methods for identifying the use of surrogates in submissions (as surrogate relationships can be implicit in economic models but not adequately presented for clinical evaluation).
Generate a curated list of methodologies that are preferred by decision-makers, in collaboration with evaluation groups and sponsors.	1. For each method in the list, create a brief guidance paper that includes the following: a. Description of the method including links to key peer-reviewed articles b. Guidance for sponsors or evaluation groups on the presentation of the method and results in a submission or assessment report (including a checklist of what data may be required to validate the method) to ensure transparency. c. Guidance for evaluation groups on how to evaluate the results generated by a method, and how to present uncertainty and the impact of the uncertainty on risk faced by decision-makers. d. Brief explanation for the decision-making committees about how to interpret the results derived by a method. e. Brief lay explanation of the method for the benefit of patients, clinicians and the broader public. 2. Provide training and guidance to evaluation groups when adopting new methods. 3. Provide feedback to sponsors on their use and presentation of analyses based on more complex methods.
Develop an explicit qualitative value framework	1. The HTA Committee to develop, in consultation with a range of stakeholders, explicit guidance regarding the elements (beyond clinical effectiveness, cost-effectiveness, and financial impact) that the committee will consider, how they will consider them, and what impact they have on decision-making. 2. The value framework would allow enough flexibility for the deliberation process itself to add value to the decisions i.e. not be pre-weighted and scored. 3. The consideration of the value elements would need to be explicit before, during and after consideration of a technology and be transparently communicated in Public Summary Documents.

	4. Develop documentation regarding how the framework will be considered during committee deliberations and guidance explaining how sponsors could provide data to respond to additional value domains, and patients or citizens could provide submissions to respond to additional value domains. 5. Informed by published research and public consultation, develop a checklist to assist HTA decision makers to integrate equity considerations into their deliberations in a more comprehensive and systematic way. Noting that some new health technologies may have a negative impact on health equity also. This could include explicit consideration of priority populations such as First Nations peoples.
Therapies that target biomarkers (e.g. tumour agnostic cancer therapies, therapies that target particular gene alterations)	1. Develop a guideline on the assessment and appraisal of tumour agnostic therapies as outlined at 6.6.4 of the HTA Review Paper on Clinical Evaluation Methods in HTA 2. Develop a guideline on the assessment and appraisal of genomic technologies and gene therapies for HTA decisions in Australia. a. This could be for pharmacogenomic technologies only, should PBAC's remit remain as appraising medicines, vaccines, advanced therapies, and codependent technologies. Alternatively, if the Unified HTA pathway is adopted and a single HTA Committee is constituted in Australia, then it could also include genomic tests more generally (i.e. for Medicare Benefits Schedule funding decisions). b. As part of the guideline development, a Statement of Principles concerning the access and use of genomic technologies and gene therapies should be co-designed with the public. This would involve patients and clinicians but also citizens who do not have an immediate vested interest in these technologies. c. The guideline would need to be consistent with the Statement of Principles but be primarily directed at "how" the evidence should be compiled and considered. It would need to be drafted by technical experts and outline the HTA methods that could be feasibly used to inform decision-making.
Pharmacogenomic technologies	Develop a guideline on the assessment and appraisal of genomic technologies and gene therapies for HTA decisions in Australia. This could be for pharmacogenomic technologies only, should PBAC's remit remain as appraising medicines, vaccines, Advanced Therapies and codependent technologies. Alternatively, if the unified HTA pathway is adopted and a single HTA Committee is constituted in Australia, then it could also include genomic tests more generally (i.e. for MBS funding decisions). As part of the guideline development, a Statement of Principles concerning the access and use of genomic technologies and gene therapies should be co-designed with the public. This would involve patients and clinicians but also people who do not have an immediate vested interest in these technologies. The guideline would need to be consistent with the Statement of Principles but be primarily directed at "how" the evidence should be compiled and considered. It

	would need to be drafted by technical experts and outline the HTA methods that could be feasibly used to inform decision-making.

3.3. Economic evaluation

Selection of the comparator	1. Develop guidelines to distinguish between the selection of comparator for submissions claiming superiority and to submissions claiming non-inferiority to make clear which comparator should be selected when there are multiple potential comparators. 2. In line with other options included to calibrate the methods and level of appraisal to the level of risk and clinical need / benefit of submissions, investigate situations where it may be appropriate to move away from the current method/s used in the application of this interpretation. a. This could include a mechanism to differentiate different type of cost-minimisation submissions based on their proportional benefit. b. Any alternative consideration would require explicit consideration of the opportunity cost and budget implications relative to the base case of the status quo. *Note: These considerations will include downstream consequences for budget impacts noting that Australia does not have policies that encourage the use of older medicines that remain as comparatively effective and safe as more recently listed alternatives and have lower prices. This results in a market share erosion of older, lower priced medicines.*
Valuing of long-term benefits	Noting the PBAC's July 2022 recommendation as follows: *"The PBAC did not recommend a stand-alone change to the base-case discount rate in its Guidelines. The PBAC recommended that, given the range of factors, in addition to the discount rate, that contribute to the assessed value of a medicine or vaccine, any policy decision on a general reduction in the standard base-case discount rate for health interventions should be assessed alongside other relevant factors in decision-making as part of the broader HTA review.* *The PBAC recommended that should the Government make a broader policy decision to change the standard base-case discount rate for economic evaluations of health interventions after considering cross-portfolio implications and the HTA Review:* • *the base-case discount rate should be no lower than 3.5% - 4% per year* • *approaches for evaluating economic uncertainty arising from value attributed to future and extrapolated benefits be adjusted to ensure the uncertainty of future costs and benefits is fully captured and considered in decision-making* • *equal discount rates for costs and health outcomes should be maintained, consistent with most common international practice* • *a mandatory 5% discount rate sensitivity analysis would need to be conducted for purpose of being explicit about the impact on opportunity cost and budget, and to ensure consistency with prior decisions by allowing advisory committees to compare ICERs for new listing requests with previously considered items based".*

	Develop modelling of the aggregate impact of the HTA Review recommendations and include different scenarios of varying the discount rate for various different technologies (in particular health technologies including those that have high upfront costs and benefits that accrue over a long period of time such as vaccines and gene therapies) to inform further consideration of any changes to the discount rate. Noting that there are circumstances where it may be reasonable to have an alternative (lower) discount rate for some therapies and in some circumstances. Measurement outcomes of the modelling should include overarching impacts to the budget and consider changes to variables such as the ICER that may require adjustment as a result of any considered change to the base case discount rate. Additionally, this should include explicit consideration of the opportunity cost and budget impacts for any change relative to the status quo.
Valuing overall	Conduct workshops to understand if and where it may be reasonable for HTA committees to accept higher prices for health technologies including: a. in what circumstances b. for what benefit c. how much greater cost would be reasonable to secure that benefit d. how confident do we need to be that we will be securing that benefit e. what measures would be appropriate to offset the higher costs over a product's lifecycle. To ensure the sentiment captured through the workshops are representative of the Australian population, workshops / consultation should include a population representative sample (including representation of key stakeholder groups) and ensure measurement is free from selection bias. Workshops could also be assisted through use of the explicit qualitative value framework proposed above (see Develop an explicit qualitative value framework).

Section 3 – Overall summary

There is support for the suggestions outlined in Option 3.1, with less discussion broadly across the submissions and minor points of difference identified between stakeholder groups. There is, however, a great deal of comment about the proposals in both 3.2 and 3.3. Both clinical evaluation and economic evaluation are talked about in depth throughout many of the submissions, the points they raise are outlined and summarised below. Of note, there is also discussion in a number of submissions about the broader social value and environmental impacts of health technologies recommended for greater inclusion and consideration in HTA evaluation.

Those stakeholders that specifically mentioned the options under 3.1, were all very supportive, particularly of the options to increase early stakeholder participation and to increase transparency, as they believed early engagement was critical to ensuring all of those who have the potential to benefit from a new therapy were included in the process.

Overall, there was support for updates to clinical evaluation methods, especially the consideration of and greater acceptance of consumer evidence, non-traditional data, Real World Data (RWD) and Real World Evidence (RWE), which many stakeholder groups believed would assist in the evaluation of new therapies. It was supported widely that these updates to clinical evaluation will improve the current methods - which were frequently seen to be too narrow. Many also highlighted that flexibility and an increased level of comfort with residual uncertainty would be required to maintain a system that could keep pace with technology. Further guidance on how these specific reforms would be implemented and assessed was requested by a number of these groups.

There was quite a robust discussion in the submissions about economic evaluation and the proposals outlined in Option 3.3. that the full economic value may not be reflected in the price that Government is willing to pay and/or that the negotiation of the price should be undertaken separately to the HTA assessment of cost-effectiveness. There were also discussions amongst stakeholders of a desire for broader economic evaluation encompassing additional factors such as environmental impact, ethical, wellbeing and societal benefit elements.

3.1. Determination of the Population, Intervention, Comparator, Outcome

Table 38. 3.1. Determination of the Population, Intervention, Comparator, Outcome: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	86%	7%	0%	7%	14
Pharmaceutical / Medical technology company	0%	61%	33%	6%	0%	18
University or research sector	0%	0%	33%	33%	33%	3
Industry association / Peak body	0%	67%	33%	0%	0%	9
Clinician (or representative organisation)	0%	100%	0%	0%	0%	2
Consulting	0%	50%	0%	50%	0%	2
State / Territory government	0%	0%	100%	0%	0%	2
Other	0%	50%	0%	25%	25%	4

Topic 3.1. - Overall summary

Those stakeholders that specifically mentioned the options under 3.1, were all very supportive, particularly of the options to increase early stakeholder participation and to increase transparency, as they believed early engagement was critical to ensuring all of those who have the potential to benefit from a new therapy were included in the process.

Patient and Consumer Representative Groups

These groups were highly supportive of these options and believed this was another key measure that would greatly increase consumer input in the HTA. One group highlighted that further clarity on the PICO criteria was needed and a few groups also focused on the need for consumer consultation was paramount.

"Early consultation on the PICO is a key opportunity that can incorporate consumer input. This includes in understanding the population, confirming relevant comparators and providing valuable real-world evidence of outcomes that matter to patients. This would be an ideal topic where guidance could be provided to consumers and consumer organisations to assist them in providing useful input." (Mito Foundation)

"Clarity on the PICO criteria is needed, while not creating further delays. Early engagement with relevant consumer groups and expert clinicians, particularly as a result of horizon scanning is a key way that this can be accomplished." (Childhood Dementia Initiative)

"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." (MND Australia)

Pharmaceutical / Medical Technology Companies

The majority of these companies were supportive of these options. There was some slight divergence in the commentary though with one company believing that by implementing these, there was potential for accelerating the HTA process for some health technologies, whilst another company needed clarity on how this would be implemented before they could accept that it would accelerate the process.

"Gaining agreement on the appropriate PICO elements prior to HTA submission could accelerate the HTA process for some health technologies. The advice currently provided during pre-submission meetings by the DoHA is often not guided or endorsed by decision-makers, and time constraints limit the depth of discussion. Whilst pre-submission meetings are somewhat helpful, they could improve the chance of a submission successfully meeting the evaluation requirements of the PBAC by including more relevant stakeholders such as the evaluator, the ESC and PBAC discussants, as well as, when relevant, consumers with lived experience and clinicians." (AstraZeneca)

"Roche supports the options proposed with regards to increasing early stakeholder input and transparency and ensure that the PICO scoping phase identifies the patient populations that could potentially benefit from the health technology. However, it is unclear how this will be implemented and whether this will improve the HTA process and expedite access to new health technologies. Roche notes that a PICO step should largely be optional, especially in the circumstance that the sponsor has a high level of confidence in an appropriate PICO and does not believe that the PICO scoping phase will add value that outweighs the scoping time. In addition to updated guidance on health equity and priority population indications, Roche highlights the need for transparency for how this is likely to impact the decision-making process and/or outcomes." (Roche)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Although there was a very high level of support for these options, a couple of groups highlighted some concerns. One group cautioned reducing the value of the hierarchy of evidence in decision making when the funding pool is limited, whilst another commented that even though early engagement with the patient populations was very positive, they believed no amount of consultation will change the situation if the patient groups are not cost-effective.

"Overall greater consumer and stakeholder input is welcome and important especially on very significantly different technologies. However, it is to be expected that the sponsors will target the population in which it has the best evidence and which will maximise chances it will be considered cost-effective. Likewise, HTA bodies are likely to want to pay for it in those cases only. It is good to understand what wider groups of patients that consumers and clinicians may want to see covered, but if those patient groups are not cost-effective then no amount of consultation will change anything unless the HTA committee decides equity considerations (for example) trump the cost-effectiveness." (Medical Technology Association of Australia)

This association made further comments that *"there are also situations for MSAC where a pre-defined PICO process should be unnecessary because the contents of the PICO are very clear. This is usually the case if the technology is to be deployed in a well-established clinical context in which it offers improvement on the current approach. A process to develop a PICO is important when the intervention is potentially changing care pathways or applying to new patient populations."* (Medical Technology Association of Australia)

Table 39. Increased early stakeholder input – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	21%	79%	0%	14
Pharmaceutical / Medical technology company	0%	0%	22%	67%	11%	0%	18
University or research sector	0%	0%	50%	0%	0%	50%	2
Industry association / Peak body	0%	0%	0%	50%	38%	13%	8
Clinician (or representative organisation)	0%	0%	0%	50%	50%	0%	2
Consulting	0%	0%	0%	50%	50%	0%	2
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	0%	0%	25%	50%	25%	4

Patient and Consumer Representative Groups

There was broad support for early stakeholder input amongst these groups. They believed early engagement was critical to ensuring that all of those who have the potential to benefit from a new therapy were included in the process.

One patient representative group supported early input on the PICO from consumer and clinical communities *"to ensure that all the relevant patient populations who could potentially benefit from the new therapy are considered in the HTA."* (Asthma Australia)

"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" (MND Australia)

"Clarity on the PICO criteria is needed, while not creating further delays. Early engagement with relevant consumer groups and expert clinicians, particularly as a result of horizon scanning is a key way that this can be accomplished." (Childhood Dementia)

"Strongly support all measures outlined in this section. As per the National Strategic Action Plan for Rare Diseases (the Action Plan), people living with a rare disease should be consider a priority population in this context to address Priority 2.4 of the Action Plan, Ensure equitable access to best available health technology." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

A number of these companies highlighted the efficiencies that could potentially be realised with the implementation of early input of stakeholders, with a slight divergence of opinion on whether the PICO engagement step should be optional or whether it should apply to all submissions.

"Gaining agreement on the appropriate PICO elements prior to HTA submission could accelerate the HTA process for some health technologies. The advice currently provided during pre-submission meetings by the DoHA is often not guided or endorsed by decision-makers, and time constraints limit the depth of discussion. Whilst pre-submission meetings are somewhat helpful, they could improve the chance of a submission successfully meeting the evaluation requirements of the PBAC by including more relevant stakeholders such as the evaluator, the ESC and PBAC discussants, as well as, when relevant, consumers with lived experience and clinicians." (AstraZeneca)

"Earlier determination and agreement on the PICO upfront for use in submissions will make the HTA process more efficient. This should be a binding agreement, however, the MSAC model should not be adopted which creates a year long process and would further delay HTA decisions." (Alexion)

"The determination of the appropriate PICO is an important step in the development of all evidence bas guidance. A PICO ensures alignment between all stakeholders early in the process and sets the direction for the assessment of the clinical and economic assessments. This subsequently reduces the risk of submissions being rejected due to disagreements around the question at hand and therefore can decrease time for patient access. Novartis believe that the

development of a PICO (and involvement of stakeholders) should apply to all submissions, not just new molecules and or major expanded indications claiming added therapeutic value. This is necessary given the difficulties that can exist in determining the appropriate comparator(s), especially given the challenges surrounding the operation of section 101(3B) of the National Health Act 1953." (Novartis Australia)

"Roche supports the options proposed with regards to increasing early stakeholder input and transparency and ensure that the PICO scoping phase identifies the patient populations that could potentially benefit from the health technology. However, it is unclear how this will be implemented and whether this will improve the HTA process and expedite access to new health technologies. Roche notes that an [additional] PICO step should largely be optional, especially in the circumstance that the sponsor has a high level of confidence in an appropriate PICO and does not believe that the PICO scoping phase will add value that outweighs the scoping time. In addition to updated guidance on health equity and priority population indications, Roche highlights the need for transparency for how this is likely to impact the decision-making process and/or outcomes." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These groups were very supportive of this option and highlighted this as another area of opportunity to incorporate stakeholder feedback into the HTA system. One group warned that this could be counterproductive if implemented, as it could increase the time taken for patients to access new therapies, as currently sponsors can nominate their own PICO and only incorporate stakeholder input if it is needed, for example where there are clinical trials, stakeholder input may not be required. One research association also argued that environmental impacts should be included in the PICO.

"Overall greater consumer and stakeholder input is welcome and important especially on very significantly different technologies. However, it is to be expected that the sponsors will target the population in which it has the best evidence, and which will maximise chances it will be considered cost-effective. Likewise, HTA bodies are likely to want to pay for it in those cases only. It is good to understand what wider groups of patients that consumers and clinicians may want to see covered, but if those patient groups are not cost-effective then no amount of consultation will change anything unless the HTA committee decides equity considerations (for example) trump the cost-effectiveness." (Medical Technology Association)

"Early consultation on the PICO is a key opportunity that can incorporate consumer input. This includes in understanding the population, confirming relevant comparators and providing valuable real-world evidence of outcomes that matter to patients. This would be an ideal topic where guidance could be provided to consumers and consumer organisations to assist them in providing useful input." (MITO Foundation)

"A technology's potential impact on the environment (e.g. carbon emissions) should also be included in the PICO - outcomes section." (Health Services Research Association)

Table 40. Increased transparency for stakeholders – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	21%	79%	0%	14
Pharmaceutical / Medical technology company	0%	0%	17%	72%	11%	0%	18
University or research sector	0%	50%	50%	0%	0%	0%	2
Industry association / Peak body	0%	0%	0%	50%	38%	13%	8
Clinician (or representative organisation)	0%	0%	0%	50%	50%	0%	2
Consulting	0%	0%	50%	0%	50%	0%	2
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	25%	0%	25%	50%	0%	4

Patient and Consumer Representative Groups

These groups were very supportive of increased transparency for stakeholders. These groups emphasised the benefits of early input, transparency and involvement of stakeholders and how these measures had the potential to add robustness to the assessments and to give organisations sufficient time to prepare any input required of them.

"Increased early input on the PICO from patient and clinician communities to ensure all relevant patient populations that could potentially benefit from the new therapy are considered in the HTA, and to identify issues that may impact implementation early to be to be addressed (for new drugs or major expanded indications claiming added therapeutic value)." (Cell and Gene Catalyst, AusBiotech)

"CCA support increased early input on the PICO from patient and clinician communities to ensure all relevant patient populations that could potentially benefit from the new therapy are considered in the HTA, and to identify issues that may impact implementation early to be addressed (for new drugs or major expanded indications claiming added therapeutic value)." (Crohn's and Colitis Australia)

"This is vital but must be done in a way that does not delay access or add time to the HTA process. This may require very early engagement of relevant consumer organisations to give them time to prepare the input required. The assistance of the Consumer Evidence and Engagement Unit or a similar team within the Department of Health may play an important role in this." (Haemophilia Foundation Australia)

"Strongly support this and believe it will lead to more robust assessments in the case of rare disease technologies and ensure that decisions respond best to unmet need in the patient population." (Rare Voices Australia)

"Essential there is explicit framework for involvement of consumers from the outset. Consumers representing specific diseases will be a high value add to consultations in relation to this section." (NeuroEndocrine Cancer Australia)

"CHF enthusiastically supports increased early input on the PICO from consumers and clinician communities. This will ensure that all relevant patient populations that would benefit from technologies are considered in the HTA process." (Consumers Health Forum Australia)

Pharmaceutical / Medical Technology Companies

Broad support and encouragement from these companies is expressed with one comment in regard to the management of stakeholder expectations on their input influencing the use of a technology outside the trialled evidence base. Other company proposed this option could be extended to genomic technologies.

"We are supportive of this recommendation and we would suggest to extend its application to Genomic technologies." (Illumina)

"Generation of a PICO early ensures that the correct populations are identified, the appropriate comparators are chosen and the outcomes are defined. This submission is then generated based on these correct factors. Inputs from stakeholders including patients and clinicians will ensure that the interest of these stakeholders will be included in the development of the PICO. This consultation will also improve transparency of the process." (Pfizer)

"As noted in the Options paper, there can be sometimes a desire for a technology to be used outside the trialled evidence base for a multitude of reasons. Roche believes that sufficient context be provided to stakeholders providing input, including that it is unlikely that a specific evidence generation package will be developed specifically for Australia where it differs from the PICO informed by the trialled evidence base. This means that there needs to be considerations about the realistic level of evidence available to answer specific questions in the PICO process." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Again, there was broad support from these groups for these options and it was highlighted that this option allowed HTA assessments to become more patient-centred, whilst one university requested more detail to be able to assess the impact of clinician and patient input into the PICO process.

"By incorporating early stakeholder input into the determination of the PICO, HTA evaluations become more patient-centred, and clinically relevant, reflective of the diverse needs and perspectives of the patient populations they aim to serve." (Society of Pharmacists of Australia)

"I think this would be worthwhile. However, I think we can still have increased early stakeholder input by updating the PBAC guidelines so that submissions request direct input from stakeholders (as opposed to setting up a separate process to elicit it)." (Shawview Consulting)

Table 41. Updated guidance – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	29%	71%	0%	14
Pharmaceutical / Medical technology company	0%	0%	17%	78%	0%	6%	18
University or research sector	0%	0%	50%	50%	0%	0%	2
Industry association / Peak body	0%	0%	0%	50%	38%	13%	8
Clinician (or representative organisation)	0%	0%	0%	50%	50%	0%	2
Consulting	0%	0%	50%	0%	50%	0%	2
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	0%	0%	50%	25%	25%	4

Patient and Consumer Representative Groups

This option was supported by these groups with an emphasis on the need for explicit consideration of health equity and priority populations for new treatments.

"Supported, early stakeholder input to the PICO will assist with determination of a suitable comparator that is reflective of the Australian standards and practice. Particular note should be made of guidance regarding validation of surrogate endpoints, as these may be disease and treatment specific." (Australasian Leukemia and Lymphoma Group)

Another organisation *"welcomes the new and explicit requirement to consider equity and priority populations for new technologies"* but they have requested more detail in regard to how this would influence decision-making in practice. They also emphasised that they were *"very keen to understand if this measure would directly help to address the longstanding issue relating to the lack of paediatric medicines in Australia.*" (Asthma Australia)

"APAA support updated guidance to require the explicit consideration of health equity and priority populations for new treatments. These populations should specifically include patient populations that can no longer benefit from the comparator classes of drugs due to either contraindications or prior loss of response." (Australian Patient Advocacy Alliance)

"CCA support updated guidance to require the explicit consideration of health equity and priority populations for new treatments. These populations should specifically include patient population that can no longer benefit from the comparator classes of drugs due to either contraindications or prior loss of response. This would allow subgroup analyses of for instance anti-TNF experienced patients in trials. CCA Support the ability to use real world evidence in submission because sponsors will never do head-to-head comparisons of new agents against all agents in class, but post marketing evidence from clinicians is highly influential to practice and should be represented in the funding decisions." (Crohn's and Colitis Australia)

"This can be prepared in advance alongside horizon scanning so that expert clinicians and consumer organisations can be prepared to contribute in a timely way." (Mito Foundation)

"We support updated guidance that requires explicit consideration of health equity and priority populations for new treatments. We propose that, in line the National Strategic Action Plan for Rare Diseases and in recognition of ongoing inequities in access to health technologies experienced by people living with a rare disease, that people living with a rare disease be recognised as a priority population for the purpose of HTA." (Rare Voices Australia)

"The previous two options must be considered applying an intersectional lens. There must be explicit consideration of health equity and priority populations, which must be given a voice. This includes (but is not limited to) First Nation Peoples Culturally and Linguistically Diverse Communities, the LGBTQIA+ community, people with experience of mental health issues, and people living with disabilities." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

These options were also supported and welcomed by Pharmaceutical / Medical Technology Companies.

"*Roche supports updated guidance to require the explicit consideration of health equity and priority populations for new treatments, and additional guidance for when and how PICO is to be developed."* (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Broad support from these stakeholders' groups as well, one university mentioned the need for updated guidance to measure the impact of health equity of interventions.

"Updating guidance to consider equity and priority populations and providing additional guidance on PICO development strengthens the patient-centeredness and relevance of HTA evaluations." (Society of Hospital Pharmacists of Australia)

"Updated guidance is required to measure the magnitude of the impact on health equity of interventions, explicitly and systematically, to ensure that funding decisions do not increase health inequalities and, where possible, reduce health inequalities for priority populations such as First Nations Australians. Potential health inequalities are rarely quantified or if considered are usually qualitative in nature. The type of equity information would vary, and quantitative analysis might focus on pre-existing health inequalities rather than expected impacts of interventions on health inequity. This makes comparing the health equity impact of different interventions difficult. Distributional cost-effectiveness analysis (DCEA) is an economic method that can quantify the population distribution of expected health benefits of interventions by Indigenous and non-Indigenous status in quality-adjusted life years (QALYs). Importantly, this method enables the comparison of the impact on health equity across various interventions." (Deakin University)

"I am not convinced that our submissions to PBAC are routinely missing information "of importance to patients and clinicians (e.g. for HATV/HUCN reasons)". I think these important things can get lost amongst all the technical considerations of a submission. However, they should always be there." (Shawview Consulting)

One government stakeholder suggested that while increased flexibility and capacity to incorporate non-standard evidence is useful, this still needs to occur in some form of standardised framework.

3.2. Clinical Evaluation Methods

Table 42. 3.2. Clinical Evaluation Methods: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	79%	11%	0%	11%	19
Pharmaceutical / Medical technology company	0%	29%	47%	24%	0%	17
University or research sector	0%	17%	50%	17%	17%	6
Industry association / Peak body	0%	67%	33%	0%	0%	9
Clinician (or representative organisation)	0%	67%	33%	0%	0%	3
Consulting	0%	0%	50%	0%	50%	2
State / Territory government	0%	0%	100%	0%	0%	1
Other	0%	40%	20%	20%	20%	5

Overall, there is support for updates to clinical evaluation methods, especially the consideration of and greater acceptance of consumer evidence, non-traditional data, Real World Data (RWD) and Real World Evidence (RWE), which many stakeholder groups believed would assist in the evaluation of new therapies. It was supported widely that these updates to clinical evaluation will improve the current methods, which were seen to be too narrow and to ensure these assessments can keep pace with technology. Several stakeholders noted a focus on flexibility and an increased level of comfort with residual uncertainty would be required to maintain a system that could keep pace with technology. Further guidance on how these will be implemented and assessed was requested by a number of these groups.

Patient and Consumer Representative Groups

The majority of these stakeholder groups supported the reform options to clinical evaluation, emphasising the need for a system that would be flexible enough to incorporate new developments and highly-specialised therapies.

"The paper referenced in the options paper outlines multiple options for each of the different types of assessment. In developing this, we must ensure that information is accessible (and transparent) to stakeholders, allowing them to make informed decisions and submissions. This process should additionally be informed by expert consumer guidance. It is overall very promising that the options consider the use of non-traditional data and RWE/RWD in the HTA. This especially should consider the expertise consumers can bring to the table. The explicit

qualitative value framework is also very promising and has the potential to improve decisions. This framework should make special considerations for rare/ultra-rare disease communities that often have limited access to high-cost, highly specialised therapies." (MITO Foundation)

"As we find new ways of measuring disease outcomes and progression in MND it is critical we have an assessment framework that is flexible to incorporate these developments. Similarly, as we develop more robust biomarkers, these also must be included in assessment considerations." (MND Australia)

"We agree that HTA assessments should allow more flexibility in the evidence base, and greater acceptance of non-randomised evidence, the role of RWD and surrogate outcome measures. The inclusion of consumer evidence and RWE evidence will be fundamental to deliver access to therapies for rare conditions and for underrepresented populations." (Childhood Dementia Initiative)

"NAA members are particularly interested in clarity and equity in how high unmet clinical need (HUCN) are defined in the implementation of these reforms. There are many rare diseases among NAA members, with no or limited treatment options/therapeutics, and there is a risk that the new HUCN criteria will prioritise larger cohorts." (Neurological Alliance Australia)

"Essential this is informed by consumer expertise and consultation. Essential there is funding to allow inclusion of all stakeholders, including expert clinical stakeholders to be involved from the outset." (NeuroEndocrine Cancer Australia)

"Current clinical evaluation methods for HTA - particularly for assessing medicines for inclusion on the PBS - is narrow and rudimentary, focusing on the lowest common denominator and taking a 'one-size-fits-all' approach at the expense of people with complex, uncommon and heterogeneous diseases. Clinical evaluation methods need to be updated so that committees can make an informed assessment of the economic costs and benefits of funding health technologies, despite the complexity of diseases that they have been designed to treat." (Anonymous submission)

Pharmaceutical / Medical Technology Companies

The majority of these companies supported these options, they expressed the same focus as some of the patient representative groups for flexibility in the assessment process. They also wanted clarification that these changes will ultimately lead to the PBAC being more comfortable with the outputs of these methods and the residual uncertainty. There was also some comment that the reforms could have been more progressive and gone further.

"The proposed options to update methods associated with nonrandomised and observational evidence, surrogate endpoints, control group creation, treatment switching and having a list of curated methods are all reasonable, if used to determine the most likely treatment effect. However, the lack of an effective risk sharing framework in Australia often results in a conservative approach to managing data uncertainty. This approach can undervalue medical innovation and disincentivises the rapid introduction of new health technologies to Australia. Therefore, to achieve the intended outcomes of the Review, robust clinical evaluation methods and broad utilisation of data sources to support evidence of clinical effectiveness must be accompanied by an effective framework for managing the risk of uncertainty" (AstraZeneca)

"Alexion supports greater flexibility in the assessment of nonrandomized and observational evidence to support the clinical and safety claims proposed in a HTA submission. However, the PBAC guidelines need to accommodate and maintain flexibility in assessing clinical data in the context they are presented (i.e. literature based submissions). In instances where an indirect treatment comparison (ITC) is required, the sponsor should have the ability to present any ITC methodology (I.e. Bucher, Match adjusted or stimulated treatment comparison) as long as the approach is justified. The PBAC guidelines need to permit flexibility especially when assessing technologies that target rare and ultra-rare diseases as its often impossible to design a perfect trial due to ethical and sampling considerations. This flexibility also needs to flow into the economic evaluation." (Alexion)

"The review paper does not address whether following the completion of all this work, and application of this by sponsors, these changes will ultimately lead to the PBAC being more comfortable with the outputs of these methods and the residual uncertainty. Increased levels of comfort with the residual uncertainty following the application of contemporaneous methodologies would lead to faster access for patients at a reasonable and appropriate value for the medicines industry. Without that increased acceptance there is little benefit in further adapting the methods at hand." (Novartis Australia)

"BMSA acknowledges the proposed options to review many of the technical methods used in HTA in Australia including the need to revise and update guidance specific to the use of indirect treatment comparison (ITC), non-randomised studies, real world evidence (RWE), surrogate outcomes, value assessment and dealing with uncertainty in HTA submissions. BMSA supports the need for revision of technical methods and updated guidance and in doing so, requests that all stakeholders are involved in finalizing the changes and that they are implemented as a priority." (Bristol Myers Squibb Australia)

"Overall, updated guidance is welcomed but the updated acceptable methodology could be more ambitious and more progressive regarding the acceptance of novel methodologies for RWD and indirect treatment comparisons, in line with other comparable jurisdictions. There is still lack of clarity on which methodologies would be considered acceptable and in which cases, and it is unclear how the HTA committees will be resourced to evaluate these novel statistical tools." (UCB Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was support for these options from these stakeholders but they did provide feedback in regard to where they believed there were still gaps for medical technology and genomic technologies.

"Overall further clarity and guidance on many of these issues is welcomed and will be very positive for the HTA process overall. However, owing to the fact that MedTech and digital health were specifically left out of the review, the evidence generation issues associated with these technologies was not specifically addressed. It is not only therapies for very small populations that are affected by data limitations. It also applies to technology with shorter lifecycles and smaller projected revenues, and which have increased difficulties with blinding in trials, as is typically the case for MedTech and digital health." (MedTech Association of Australia)

"A technology's impact on the environment should also be included in the clinical impact assessment. e.g. carbon emissions contributing to climate change and subsequent health issues related to heat and rising sea levels." (Health Services Research Association)

"Clinical value should be separate through a strengthened ATAGI not this process." (Immunisation Coalition)

Table 43. Overarching principles for adopting methods in Australian HTA – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	11%	61%	22%	6%	18
Pharmaceutical / Medical technology company	0%	0%	41%	47%	0%	12%	17
University or research sector	0%	0%	20%	40%	20%	20%	5
Industry association / Peak body	0%	0%	0%	67%	22%	11%	9
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	3
Consulting	0%	0%	0%	50%	0%	50%	2
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	0%	0%	0%	50%	0%	50%	4

Patient and Consumer Representative Groups

There is support for overarching principles but there was also a number of patient and consumer groups who requested further clarity, as well as detail around thresholds for uncertainty.

"More clarity is needed on data quality standards and performance indicators." (Mito Foundation)

"General agreement with overarching principles but the definition of high quality evidence needs to be clearer. This definition should be context appropriate and include definitions for high quality evidence in very small patient populations where RCTs make not be possible." (Rare Voices Australia)

"CHF is broadly supportive of the overarching principles. Principle number 8 states that "the acceptability of uncertainty in estimates may be greater in areas of high clinical need". This principle is particularly important and care must be taken to ensure that thresholds for uncertainty are clearly delineated and established as objectively as possible." (Consumers Health Forum)

Pharmaceutical / Medical Technology Companies

There is broad support from Pharmaceutical / Medical Technology Companies for overarching principles. However their questions raised about these principles concern the mention of provisional funding pathways and the ability to accept the need for greater flexibility and acceptance of non-RCT evidence.

"Roche supports the implementation of overarching principles to guide methods in Australian HTA, particularly the following points cited (p.103, Options Paper):

- *Provision of feedback to sponsors/applicants on the use and presentation analyses derived from more complex methods*
- *Acceptance of complex methods that introduce considerable uncertainty in the estimates when paired with provisional funding pathways*
- *Greater acceptability of uncertainty in estimates in areas of high clinical need (which will need to be defined so that this can be applied consistently)."* (Roche Products)

"While we can agree that clear guidance on how to use and present non-RCT data would be useful though it's more important to recognise the need for greater flexibility in the evidence base and greater acceptance by PBAC of non-RCT evidence. Without an acceptance that additional flexibility is required where RCT evidence is not available, for example diseases with small patient cohorts, there will be no improvement in time to access because for some conditions and therapies, RCT evidence will continue to be available in limited circumstances. We note the Clinical Evaluation Methods discussion paper noted that '(uncertainty) may be more acceptable if paired with provisional funding pathways'. Methods guidance alone will not improve time to access unless there is explicit guidance around the circumstances where flexibility can be applied regarding type of evidence." (Pfizer)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Again, there was general support for the principles amongst these groups. Provisional funding pathways are mentioned again here and a question about co-dependent technology assessment was raised.

"I like some of these principles more than others. But the idea of an overarching set of principles that all stakeholders understand and adhere to could represent the biggest improvement to the conduct of HTA and decision making in Australia." (THEMA Consulting)

"Welcome particularly that methods should be only as complex as required to address the problem. Not always the case now." (Medical Technology Association of Australia)

"This approach ensures consistency, transparency, and rigor in the assessment process, ultimately enhancing the reliability and credibility of HTA findings. By adhering to these principles, decision-makers can make informed choices based on robust evidence, leading to more effective allocation of healthcare resources and improved patient outcomes." (Society of Hospital Pharmacists of Australia)

"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." (Omico)

Table 44. Methods for the assessment of nonrandomised and observational evidence – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	47%	47%	6%	17
Pharmaceutical / Medical technology company	0%	6%	41%	47%	0%	6%	17
University or research sector	0%	0%	40%	40%	20%	0%	5
Industry association / Peak body	0%	0%	0%	67%	22%	11%	9
Clinician (or representative organisation)	0%	0%	0%	67%	33%	0%	3
Consulting	0%	0%	50%	0%	0%	50%	2
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	0%	0%	25%	50%	0%	25%	4

Patient and Consumer Representative Groups

There is a high level of support and a great deal of commentary for the methods of assessment of nonrandomised and observational evidence amongst these stakeholder groups. Some believed this expansion of clinical evaluation methods was a much more holistic, modern and flexible approach that could fast track access for patients. A number of patient groups saw this is a critical step and consideration for smaller patient cohorts – particularly paediatrics, where research evidence was often lacking, and they emphasised the need for these to be considered high-quality research.

"Supported, it will be important to determine what is quality real world data and its representation of the Australian environment. Support flexibility to adopt real world evidence balanced with other levels of evidence such as evidence generated from clinical trials." (Australasian Leukaemia and Lymphoma Group)

"Taking a more holistic assessment of the impacts of medicines to inform its cost effectiveness, rather than just baseline clinical outcomes based on the lowest common denominator is positive. Real-World Evidence as well as consumer experience and outcome measures as well as disease-related experience, including post market evidence, must be considered in decision-making processes." (Australian Patient Advocacy Alliance)

"BCNA particularly supports the options presented to allow fast-tracked PBS subsidies for new therapies with high unmet clinical need (HUCN) that may not have adequate RCT evidence, for a fixed time period within which RWD can be gathered and reevaluated as to the cost-

effectiveness of these therapies. BCNA notes there would need to be consideration given to navigating the event in which a therapy is removed from the PBS after this time period due to insufficient evidence." (Breast Cancer Network Australia)

"Support the ability to use real world evidence in submissions broadly and not on a restricted basis because sponsors will never do head-to-head comparisons of new agents against all agents in class, but post marketing evidence from clinicians is highly influential to clinician practice and should be represented in the funding decisions. This is important for paediatric IBD patients who need access to medications approved for adults but lack research evidence in the paediatric population. RWE is often available in Australia or in countries with well-developed health systems and should be used to support access to t medications that provide important alternative for those who have failed other treatment options. Similarly, inclusion of evidence about elderly populations, who are rarely included in IBD RCTs, is required to support quality use of medicines in this group. Currently PBS criteria restrict dosing of biological therapies. CCA supports the use of RWE to inform flexible dosing where there is a clinical benefit to the patient. Responsive systems are required to translate evidence into change in access to drugs." (Crohn's and Colitis Australia)

"This is particularly important for childhood dementia disorders where placebo controlled trials are logistically near impossible due to the scarce number of eligible patients and unethical given the invasive nature of treatment (e.g. intrathecal injection) and narrow treatment window. It is likely that a child allocated to the placebo arm of a trial will deteriorate during the trial to a point where they are no longer eligible for treatment." (Childhood Dementia Initiative)

"Generating evidence from direct randomised trials is entirely achievable for common conditions which develop and manifest in a typical manner, such as diabetes or asthma, where the clinical benefits of medicines can be easily demonstrated. But for less common and more complex conditions like lupus, which affect each patient differently, it becomes more difficult to demonstrate the clinical effectiveness of medicines, particularly where what works well for one lupus patient might not work for another. Thus, more traditional forms of evidence such as RCT are not suitable. As noted by the Reference Committee, the evidence base for health technologies and methods for assessing evidence are evolving, particularly for rare diseases. As such, nonrandomised and observational evidence should be given greater regard as part of clinical evaluation methods in order to provide patients with complex, uncommon and heterogeneous diseases with reasonable access to more effective treatments." (Anonymous submission)

"Additional methods for the use of this type of evidence is welcomed and important but the approach implies that this is not high quality evidence. A more fit-for-purpose approach would be to redefine high quality evidence in populations where RCTs are either not possible or not appropriate. It is essential that technologies for HUCN in small patient populations can be assessed equitably in Australian HTA." (Rare Voices Australia)

"CHF understands that the use of nonrandomised and observational evidence requires an in- depth assessment of the bias that might be affecting the data. In principle, CHF supports the proposed updates to the methods of assessment for such data. There is a potential that these rigorous methods might slow down the process of utilisation of non-randomised and observational evidence, and lead to a de-facto underutilisation of this evidence. Thresholds for uncertainty must be clearly demarcated. In the use of Real World Data and Real World Evidence,

consumers are concerned about privacy and data guardianship. Therefore, if consumer- generated evidence is to be used on a more consistent basis, strong systems of data safety and guardianship must be in place. Not only will this ensure that consumers feel safe releasing their data, but it will also have positive ramifications at the population level, with an increase in the quality and quantity of available data. Measures should also be put in place to prevent consumer- generated data from being used for financial gain. Consumers are adamant that while they are happy to release data for altruistic purposes, its use for financial profit is completely unacceptable. Legislators must not shy away from the challenge of ensuring that there are clauses in place preventing this from happening." (Consumers Health Forum)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies also provided broad support for these methods, with some of the companies requesting further guidance on the use of RWD and RWE. Many of these companies also wanted reassurance that these new methods would be considered high quality research and that there was enough flexibility and acceptance of these methods to ensure new technologies with a smaller research evidence base were not undervalued.

"The use of non-randomized and observational evidence, as well as RWE/ RWD for HTA purposes could be extended to other health technologies, like Genomics. The generation of RCT data are difficult for Genomic technologies, mostly because of shorter product life cycle, and therefore there is a need to recognize other sources of clinical and economic evidence to sustain the benefits of Genomics technologies going through HTA." (Illumina)

"There must be greater acceptance of non-RCT evidence in decision-making, for example real world evidence, not just more guidance on preferred methods as outlined in this option. While we generally agree with the proposed methods, without agreement that additional flexibility is required where RCT evidence is not available, for example diseases with small patient cohorts, therapies will continue to be undervalued because of uncertainty associated with available evidence. These options could add clarity on preferred methods but are not much different from the status quo in terms of allowing for greater flexibility in HTA decision making." (Pfizer)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was general support amongst these groups also, with the significance of these methods for children being raised again. Some further clarity and understanding about the use of RWD and RWE was also requested, and the focus again was on ensuring that non-RCT evidence would be considered high-quality and fit-for-purpose in some instances.

"We highlight comments in the Options Paper that many stakeholders request more flexibility in the evidence base used in HTA, including greater acceptance of non-randomised evidence. We support options relating to updated guidance on the use of non-randomised and observational evidence (including indirect comparisons, Real World Data (RWD) and Real World Evidence (RWE), other non-traditional evidence." (NACCHO)

"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." (Monash Children's Hospital)

"Very welcome in principle however under 5c it is unclear whether the 4 criteria for accepting RWE are all required or only one or some. However, the situations where this applies should not be limited to only technologies for use in a population with HUCN. Important new medical devices and digital health devices may add value but not in an area of high unmet need but cannot reasonably generate the large amount of evidence expected for a standard pharmaceutical submission." (MedTech Association of Australia)

"Supported, although it is noted that use and validity of this type of evidence will very much depend on the case/circumstances at hand (e.g. whether better quality data can be sourced). The main point to determine is whether the incremental observed effect is of such a magnitude that the probability of it occurring as a consequence solely through confounding is low. Irrespective of the methodological approach and the proposed guidance on the type of nonrandomised/observational evidence to present, the actual magnitude of effect is unlikely to be determined with precision and certainty, and this will impact on decision-making regarding the cost-effectiveness of the medicine." (Adelaide Health Technology Assessment)

Table 45. Methods for the assessment of surrogate endpoints – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	7%	40%	20%	33%	15
Pharmaceutical / Medical technology company	0%	0%	41%	53%	0%	6%	17
University or research sector	0%	0%	20%	60%	20%	0%	5
Industry association / Peak body	0%	0%	0%	75%	13%	13%	8
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	3
Consulting	0%	0%	50%	0%	0%	50%	2
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	0%	0%	0%	100%	0%	0%	2

Patient and Consumer Representative Groups

The majority of these groups believed methods of assessment of surrogate endpoints would be positive. However, there were a number who were not sure, and their level of understanding of these clinical assessment methods may be limited or not applicable to their patients or consumers.

"Surrogate endpoints can be very useful for the assessment of the co-dependent technology or testing. Strongly support the guidance process outlined." (Australasian Leukaemia and Lymphoma Group)

"Surrogates are required to be validated - why not RWE. It requires greater investment to customize specialized wearables or use expert score cards to exclude non-valid measures but these would add power to any study." (Save our Sons Duchenne Foundation)

"Surrogate endpoints are also important for childhood dementia clinical trials because measuring clinical outcomes such as cognitive performance are extremely difficult and variable in children. This means that clinical trials have to be very long and costly in order to prove a statistically significant change. Many clinical trials are being halted because the small companies that run these trials become financially unviable. If they can be approved and funded (even provisionally) based on established biomarkers, this would enable access to promising therapeutics for these children with fatal, progressive conditions. This would encourage innovation and more therapeutics to be developed for these children." (Childhood Dementia Initiative)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies were neutral or positive about the methods of assessment of surrogate endpoints. One company commented on the importance of these methods being supported by a framework and resourcing and another emphasised the significance of the approaches and guidance remaining flexible to keep pace with technological advances. There was also a comment about the data required being a hindrance to the use of surrogate.

"The data required to establish a surrogate is hindering their use and creating uncertainties in submissions. AZ agree with the Options paper that HTA bodies could facilitate the use of surrogate endpoints by curating a list of previously accepted surrogate endpoints and ensuring the methods required to validate surrogates are achievable by industry." (AstraZeneca)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These groups were also neutral or positive on assessment methods of surrogate endpoints, a couple of these groups believed that guidance on this issue was already extensive.

"Supported, although it is noted that there is a great deal of guidance on this issue in the PBAC Guidelines but it is rare that this guidance is followed by submissions." (Adelaide Health Technology Assessment)

Table 46. Generate a curated list of methodologies that are preferred by decision-makers, in collaboration with evaluation groups and sponsors – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	41%	41%	18%	17
Pharmaceutical / Medical technology company	6%	0%	41%	47%	0%	6%	17
University or research sector	0%	0%	40%	20%	40%	0%	5
Industry association / Peak body	0%	0%	0%	78%	22%	0%	9
Clinician (or representative organisation)	0%	0%	0%	67%	33%	0%	3
Consulting	0%	50%	0%	0%	0%	50%	2
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	0%	0%	50%	50%	0%	0%	4

Patient and Consumer Representative Groups

There was a great deal of support for the generation of a curated list of methodologies preferred by decision makers, they believed this would be very helpful and assist with transparency and engagement. There were suggestions that the list needed to be developed with consumers, clinicians and particular disease experts to maintain a level of flexibility to accommodate rare diseases or those with unique characteristics or populations.

"Will assist with transparency and engagement with the clinicians and consumers in the process." (Australasian Leukaemia and Lymphoma Group)

"Development of this should be done in consultation with consumers and consumer organisations." (MITO Foundation)

"Clarity on what is acceptable is valued but there should be some flexibility to accommodate populations with unique characteristics that may not be amenable to standard methodologies." (Childhood Dementia Initiative)

"It is essential that this list is developed with input from rare disease clinical and consumer experts to address the specific challenges of assessments of technologies for rare diseases." (Rare Voices Australia)

"Essential there are pathways for Health Technologies without a Sponsor." (NeuroEndocrine Cancer Australia)

"Another area that will require future consideration is the clinical criteria used to assess technology. An HbA1c check, which measures an individual's average blood glucose levels has long been the gold standard. However, Time in Range (TIR), a measurement facilitated by CGM, is fast emerging as a key indicator of improved long-term outcomes. It refers to the percentage of time a person's blood glucose levels are in a target range over the course of a day. The more time spent in range, the lower the risk of diabetes-related complications. It also highlights the length of time a person spends in hypoglycaemia and hyperglycaemia or if there is considerable glycaemic variability (the degree to which a person's blood glucose levels fluctuate). It can also be a better measure of glucose levels over time than HbA1c where haemoglobin turnover is higher than expected. This may be particularly important in assessing fitness to drive or during pregnancy among ethnic groups with an increased risk of haemoglobinopathies. Additionally, a high-level of glycaemic variability is associated with an increased risk of diabetes-related complications." (The Australian Diabetes Alliance)

"CHF acknowledges the mentioning of consumers being informed with brief, lay explanations. These explanations must not be too simplistic and must provide a clear overview of the different methodologies. There needs to be appropriate resourcing to ensure that the list is maintained and kept up to date, and that the information is available in several languages." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

The majority of Pharmaceutical / Medical Technology Companies were supportive or neutral of this option. There was mention of the list's potential assistance for areas where evidentiary deficiencies exist, and a number commented that they would like to understand how often the list would be updated and how the list was going to be developed.

"We agree with this proposal provided the list of methodologies is continuously updated, with appropriate consultation, to include new methods as they become available." (Pfizer)

"Roche supports the development of a curated list of methodologies that decision-makers prefer, as this will help provide sponsors important guidance for developing HTA submissions, especially in areas where evidentiary deficiencies exist (e.g. rare diseases, targeted and advanced therapies and genomics). However, increased flexibility and acceptance by decision- makers with regards to non-traditional data sets is critical." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was again very broad support from these groups for the development of this list, with most believed it could facilitate a more informed and efficient evaluation process. However, one consulting group highlighted they had a concern about whether this list was futureproof. A University also questioned the need for the list, with the PBAC guidelines already being quite clear on what is preferred.

"Very positive subject to implementation." (Medical Technology Association of Australia)

"This initiative ensures that decision-makers and sponsors have access to standardised information and resources, facilitating a more informed and efficient evaluation process.

Additionally, providing training and feedback mechanisms further supports the adoption and effective utilisation of these methodologies, ultimately improving the quality and reliability of HTA assessments." (Society of Hospital Pharmacists of Australia)

"The need for this was unclear. The current PBAC guidelines are explicit on what is preferred and no reason for any change to the current evidence hierarchy was provided. As noted above, the PBAC has, and will accept lower quality evidence (and MSAC has also managed with lower quality evidence for their submissions frequently) when it is recognised as the best available and will make decisions based on this. As part of the evaluation process, horizon scanning is conducted for any potentially relevant upcoming trials and results which may assist in decision making." (Institute for Health Technology, Deakin University)

"I don't necessarily like this because it isn't particularly future proof, and it encourages (maybe even mandates) potentially suboptimal methodologies for a given decision problem." (THEMA Consulting)

Table 47. Develop an explicit qualitative value framework – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	44%	50%	6%	18
Pharmaceutical / Medical technology company	0%	6%	11%	56%	22%	6%	18
University or research sector	0%	0%	50%	0%	50%	0%	4
Industry association / Peak body	0%	0%	0%	63%	38%	0%	8
Clinician (or representative organisation)	0%	0%	0%	50%	50%	0%	2
Consulting	0%	0%	0%	0%	100%	0%	2
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	0%	0%	50%	50%	0%	0%	4

There is very broad support and encouragement from the vast majority of stakeholder groups on this option. A number of these groups highlighted that this would assist the HTA process in capturing the holistic value and benefits of a new technology, including socioeconomic, with many providing comment on what they believed the framework should capture to be effective. It was also mentioned widely that the framework should be co-designed with all stakeholders and potentially be embedded through legislation.

Patient and Consumer Representative Groups

There is broad and strong support for an explicit qualitative framework, with some recommendations provided from these groups in regard to what should be included and captured in this framework.

"Framework to capture PREMS, PROMS and RWD to support consumer submissions." (Collaborative Consumer Group Response)

"Mito Foundation strongly supports the development of this framework and are excited about the opportunities that revised value frameworks might bring. Particularly:

- *the inclusion of non-health costs such as psychosocial benefits, reduction in disability, improvements in ability to work and impacts on carers;*
- *methods for assessing non-RCT data, including observational and qualitative data;*

  key considerations for consumers that should be included in this framework include impacts on non-health costs such as impact on family and carers, welfare and disability support, equity and severity of disease, clinical need (HUCN). Co-design with consumers will also introduce the value of hope and knowing in the framework." (Mito Foundation)

"This may help to more appropriately and fairly cater for people with complex and less common diseases, which affect each patient differently (like lupus), and thus where traditional clinical evidence (such as RCT) may be unsuitable." (Anonymous submission)

"Strongly support the develop of this framework and as per the Clinical Methods in HTA Evaluation expert paper key considerations for consumers should be included, such as:

- *Family and carer spillovers*
- *Equity*
- *Severity of disease*
- *Value of knowing*
- *Availability of alternatives (HUCN)*
- *Productivity*
- *Value of hope*

Additionally, Lakdawalla et al (2018) in their paper Defining Elements of Value in Healthcare propose a real option value is proposed for use in life extending technologies for conditions with high mortality rates. This value should be considered when developing a values framework." (Rare Voices Australia)

"In addition to just looking at cost effectiveness across a trial population, look at the health economic modelling of the patient groups that have a higher clinical need or fewer available therapies or are excluded from clinical trials - the socioeconomic effects of failure to list a new therapeutic class are far greater on these patients than on treatment naive patients that make up to bulk of the registration studies for earlier therapies that are now used as the reference comparator. Indirect costs like time out of work/role should be included in the health economic modelling." (Crohn's and Colitis Australia)

"Essential there is explicit framework for involvement of consumers from the outset, along with the appropriate levels of resourcing to support their inclusion." (NeuroEndocrine Cancer Australia)

"CHF supports the explicit and systematic use of qualitative evidence during committee deliberations. It is through qualitative evidence that consumers can demonstrate the broader social benefits, cost efficiencies, and unintended financial impacts that technologies will produce. This allows the HTA process to elevate itself from a "dollars and cents" view of health, and provide recommendations that consider broader economic and social impacts. CHF supports the development of a checklist to assist decision makers to integrate equity considerations. There must be enough funding to periodically update the list, to ensure it remains current." (Consumers Health Forum of Australia)

"This is really important to provide clarity and certainty about how value is understood and included as part of the HTA process. It is clear that currently different stakeholders have differing views on what this means which also translates into the evidence that is collected and provided. This would need to be a co-designed and developed framework." (Genetic Support Network of Victoria

Pharmaceutical / Medical Technology Companies

Many of these companies provided strong support for the development of this framework and believed that the broader assessment of value would be very beneficial.

"An explicit qualitative framework (MCDA or equivalent) for rare diseases could support the achievement of faster access for rare disease medicines." (Takeda)

"This is really important to provide clarity and certainty about how value is understood and included as part of the HTA process. It is clear that currently different stakeholders have differing views on what this means which also translates into the evidence that is collected and provided. This would need to be a co-designed and developed framework." (Genetic Support Network of Victoria)

"Feedback from stakeholders in Consultation 1 highlighted that greater structure and transparency in how contextual factors such as severity, rarity and equity are incorporated into funding recommendations are required. AZ concurs with this observation. The methods expert paper outlined that a range of methods have been developed for explicitly considering multiple criteria such as quantitative multiple criterion decision analysis (MCDA) and Distributional Cost-Effectiveness Analysis (DCEA), however, such approaches may require considerable resources to generate and delay the time to access. AZ concurs with the Options paper that a more structured approach as to how non-economic HTA elements is considered by PBAC is required. The option to develop an explicit qualitative value framework and reported in Public Summary Documents would have a positive impact. The elements included in the framework should be co-designed with stakeholders." (AstraZeneca)

"It is essential this work is done very carefully and needs to reflect community and stakeholder values. This should not be developed by the PBAC as they are not the policy making committee." (Amgen)

"The qualitative value framework will assist Australia's HTA system in delivering on society's needs and preferences for medicines. It should include criteria for situations where second-order effects on patients and their caregivers, such as social welfare and carer impacts, and productivity benefits should be included in the HTA assessment process, including workable

methodologies for the transparent inclusion of second-order effects or patient benefits, in a way that supports early and equitable access. This includes quantification of second-order effects in base case economic evaluations. This process to develop the framework should be elevated to an independent policy initiative led by a coalition of all relevant stakeholders, and not run by the HTA Committee. Once finalised the value framework should be embedded in legislation to ensure there is no conflict with the NHA." (Eli Lilly Australia)

"A qualitative value framework is essential, and this requires clear criteria, including how qualitative factors can influence decisions. It is important that it comprehensively reflects the value of health technologies in addressing the needs and preferences of society. For example, currently vaccines are consistently undervalued in Australia because of high discount rates, systematically low ICERs and no consideration of broader benefits to society." (Pfizer)

"AbbVie is supportive of the development of a qualitative value framework to facilitate greater transparency and consistency around how evidence beyond clinical effectiveness, cost-effectiveness and budget impact is factored into HTA decision making. It is a crucial component in taking a holistic approach to value assessment and ensuring that Australia's HTA system is aligned with broader societal preferences regarding spending on health care and access to new health technologies. The value framework must be developed independently by a coalition of all relevant stakeholders, separate from the PBAC, in order to ensure objectivity and alignment to patient and broader societal values. Consultation across a broad range of stakeholders during development of the framework will be essential to ensure all potential value domains are considered and represented fairly." (AbbVie)

"It would be beneficial to understand the role that industry stakeholders could have on the development of this framework, since some elements such as the value of innovation or R&D are only important for a fraction of the stakeholders. Further, elements that are important to patients and the society (return to work, convenience, value of hope), must be included in the overall evaluation, even if they are not included in the ICER calculations." – UCB Australia

"Roche supports the development of an explicit qualitative value framework in consultation with stakeholders. Roche notes that this should be run as an independent policy initiative, and independently of the HTA committee, to incorporate broad perspectives from all relevant stakeholders to develop the framework. Roche notes that a reasonable starting point to commence are the Elements of Value specified in the Defining Elements of Value in Health Care A Health Economics Approach: An ISPOR Special Task Force Report (Figure 1, Lakdawalla 2018, https://www.valueinhealthjournal.com/article/S1098-3015(17)33892-5/fulltext). It would be anticipated that this value framework includes criteria for circumstances where second-order effects on patients and their caregivers, such as social welfare and carer impacts, and productivity benefits are included in the HTA assessment process (and be acceptable and incorporated into any base case analyses). This includes workable qualitative and/or quantitative methodologies for the transparent inclusion of second-order effects or patient benefits. This would be welcomed as an important first step in recognising the broader value of new health technologies." (Roche Products)

"The process to develop a value framework should be elevated to an independent policy initiative led by a coalition of all relevant stakeholders, and not run by the HTA Committee. Once finalised the value framework should be embedded in legislation to ensure there is no conflict with the NHA. Criteria should be explicit and should include second-order effects." (A.Menarini Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was a very positive and supportive response to the option to develop a qualitative framework. These groups were particularly positive about the potential for further flexibility, expansion of factors considered and increased transparency of the decision making process. There were some further suggestions about elevating the development of this framework to an independent group of representatives and once developed, ensuring this is formally embedded through legislation.

"Supported, particularly this statement "The value framework would allow enough flexibility for the deliberation process itself to add value to the decisions i.e. not be pre-weighted and scored."" (Adelaide Health Technology Assessment)

"I am very positive about this. I can see that each submission could be assessed against a matrix/checklist of qualitative values (e.g.: clinical need: low, moderate, high, very high; magnitude of effect: low, moderate etc; equity issues, first nations, life threatening etc). Assessing submissions against this checklist (which I assume would be the same for all submissions?) will add an element of objectivity/reproducibility/transparency to the subjective "other factors" which the PBAC have to contend with in every single submission. Also, I would not necessarily limit this explicit qualitative value framework to issues "beyond clinical effectiveness, cost-effectiveness, and financial impact" because each of those assessments have qualitative values embedded within them (e.g.: does the evidence support a 10 year extrapolation or a 5 year extrapolation, quantitative in effect, but this is a qualitatively judgement in practice). I can understand the reluctance to score these qualitative values so as to maintain flexibility in decision making. However, I don't think scoring (or categorising) submissions within a qualitative framework necessarily compromises this flexibility. There will still be flexibility in deciding which of the subjective categories each submission is categorised as. However, this categorisation (or scoring) will provide future sponsors with a transparent reference point to know what they can expect if their submission is in a similar situation." (Shawview Consulting)

"SHPA is very supportive of the need to develop an explicit qualitative value framework in consultation with stakeholders. SHPA believes that value should recognise the clinical, social, and financial value of approving or subsiding a health technology to enable access to patients requiring it, compared to not approving or subsidising it i.e., what are the implications of disease progression on a range of factors including, mental health, family life, loss of work, and hospitalisation." (Society of Hospital Pharmacists of Australia)

"Not only is an explicit qualitative framework required, a checklist of quantitative estimates of the impact of interventions on health inequalities is needed. The checklist would allow health economists to critically appraise quantitative estimates produced by external parties of the health equity impacts of interventions on priority populations such as First Nations people. Aboriginal and Torres Strait Islander input is required in the development of an explicit framework. A checklist for Critical Appraisal of Health Inequality Impact Estimates has been developed in the UK by expert health economists. The checklist contains both quantitative and qualitative equity considerations that are important for HTA decision-making. A similar checklist should be developed and tested for the Australian context in combination with health equity impact calculators that can quickly assess and check the likely direction and size of health inequality impacts. A copy of the checklist is in the reference below." (Deakin University)

Table 48. Therapies that target biomarkers (e.g. tumour agnostic cancer therapies, therapies that target particular gene alterations) – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	12%	35%	29%	24%	17
Pharmaceutical / Medical technology company	0%	0%	24%	65%	0%	12%	17
University or research sector	0%	0%	40%	40%	0%	20%	5
Industry association / Peak body	0%	0%	11%	78%	11%	0%	9
Clinician (or representative organisation)	0%	0%	33%	33%	33%	0%	3
Consulting	0%	0%	50%	0%	0%	50%	2
State / Territory government	0%	0%	0%	100%	0%	0%	1
Other	0%	0%	0%	75%	0%	25%	4

Patient and Consumer Representative Groups

A number of these groups who supported these options have highlighted an urgent need for these guidelines as this currently represents a large gap. There was also a concern raised around privacy of genetic information.

"This is a huge gap at the moment." (Genetic Support Network of Victoria)

"There is hesitancy among the community to have compulsory genetic testing linked to access to drugs. This is primarily related to privacy of genetic information and risk of disclosure." (Crohn's and Colitis Australia)

"Our concerns for this option centre on the challenges of working with the general public to gauge value when most do not have lived experience of the health condition being considered. How can expert consumers and innovative consultation approaches be used to help the general public play a role in determining value?" (Mito Foundation)

"Support the guidelines and the proposed unified HTA pathway. It is important to note that there will still be need for review for tumour specific biomarker therapies i.e. not all biomarkers across tumour groups work with universal effectiveness and it would be unwise to reject a therapy prematurely. The curation of generic variants is complex, so approvals must be in line with current evidence where there is data to support the therapy and the target biomarker." (Australasian Leukaemia and Lymphoma Group)

"CHF supports option 2b, which calls for the development of a Statement of Principles concerning the access and use of genomic technologies and gene therapies that is developed in co-design with consumers, clinicians, and the broader public." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

There was general support for these options. Some of the companies commented on the need for co-design and further funding for access and support for comprehensive genomic testing for a broader range of cancers.

"AZ agrees with the Options paper that linking the cost of a test to a single medicine may no longer be possible as innovation in diagnostics is providing more information beyond targeting a treatment to a biomarker). AZ is also a proponent for funded access to comprehensive screening and genomic testing for a broader range of cancers beyond the most common cancers. This includes investment in broad and routine screening for more cancer types and increased access to genomic testing through a coordinated national approach similar to the National genomic Test Directory in the UK (operated by the NHS) - as discussed at the recent Senate Inquiry into equitable access to diagnosis and treatment for individuals with rare and less common cancers. Such an approach could facilitate a de-coupling of the HTA evaluation of medicine and test to increase efficiency in the HTA evaluation process." (AstraZeneca)

"We support the development of guidelines on the assessment and appraisal of tumour agnostic therapies, should also include guideline on the assessment of associated genomic technologies. However, there is a need to better define what is meant by 'pharmacogenomic technologies'. Regarding the development of guidelines, the options state that this would involve patients and clinicians and citizens. There would be a need to include industry as who are instrumental in the access of Genomic technologies and can provide a breadth of expertise for the assessment of these technologies. (Illumina)

This company also went on to explain *"however, the development of guidelines will not suffice to grant better access for Genomics technologies. As pointed out in the New Frontiers report, a recommendation was made that 'The independent Health Technology Assessment Review reassess relevant aspects of the Health Technology Assessment process to ensure there are future pathways for treatments and therapies that do not fit neatly into the current system such as precision medicines'. It was further noted that 'The Committee is of the clear view that precision medicine approval pathways will require a different application assessment than current approaches designed for treatments for common conditions, with large data sets and comparative evaluations'. Despite the agreement from the Government, we haven't see any progress on the development of fit-for-purpose access pathways for precision medicine and Genomics technologies in particular"* (Illumina).

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was again broad support amongst these groups, with insight provided on the current co-dependency of therapeutic approvals on companion diagnostics.

"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 tumour 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." (Omico Foundation)

This foundation went on to explain that *"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."* (Omico Foundation)

"I don't have a particular strong view on this other than to say that good quality HTA methods can overcome the kinds of issues described in the paper (poor sample size, heterogeneous populations, wrong comparator). The issues might be more concentrated in situations like this, but they are far from unique to therapies that target biomarkers." (THEMA Consulting)

Table 49. Pharmacogenomic technologies – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	18%	29%	29%	24%	17
Pharmaceutical / Medical technology company	0%	6%	18%	65%	0%	12%	17
University or research sector	0%	0%	60%	0%	0%	40%	5
Industry association / Peak body	0%	0%	11%	67%	11%	11%	9
Clinician (or representative organisation)	0%	0%	33%	0%	33%	33%	3
Consulting	0%	0%	50%	0%	0%	50%	2
State / Territory government	0%	0%	0%	100%	0%	0%	1
Other	0%	0%	25%	50%	0%	25%	4

Patient and Consumer Representative Groups

There was a smaller cohort who responded and supported the pharmacogenomic technology options. A number of these groups didn't know if there would be an impact on their organisation.

The Collaborative Consumer Group Response submission highlighted a gap that currently exists *"in funding pathways for genomic/pharmacogenomic technologies that currently fall between Commonwealth funding and State funding (e.g. Trio sequencing where one person is an inpatient and the other 2 people requiring testing are outpatients)."* (Collaborative Consumer Group Response)

"Codependent review is supported, consideration to value for money and longevity of the technology should be part of the horizon scanning work to ensure that Australia continues to

evolve and adopt best practice and technologies." (Australasian Leukaemia and Lymphoma Group)

"We note the proposal to develop a statement of principles concerning the access and use of genomic technologies and gene therapies through clinician/patient co-design and that this should include people who do not have an immediate vested interest in these technologies. We agree that it is important that the process includes a more objective viewpoint. However, in rare diseases these are highly specialised situations and these participants would need careful briefing so that they could understand the patient experiences and the impact on their quality of life and be able to put the value of the new technology in a perspective that is comparative to the existing therapies and patient outcomes." (Haemophilia Foundation Australia)

"Guidelines can help to address existing issues related to the funding gaps between the states/territories and the Commonwealth. These guidelines should also cover diagnostic genomic testing in the absence of a treatment. This is an important HTA decision and consistency between how decisions are made with and without a treatment is important. The options paper describes co-designing with the public, in particular those who do not have an immediate vested interest in these technologies. While we understand the need to balance passionate consumer input in these processes, any public engagement, particularly of rare conditions, must utilise methods that include the education of members of the public, such as the briefing steps within a citizens jury." (Mito Foundation)

"This is a critical option to address current barriers to funding of genomic technologies that fall between commonwealth and state funding mechanisms e.g. For example, the Australian Genomics acute care genomics project that provided rapid trio whole genome sequencing (WGS) for infants and their parents in hospital currently has no clear funding pathways in current HTA/National Health Reform Agreement 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." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

There was support, but not a great deal of commentary around this option from Pharmaceutical and/or Medical Technology Companies.

"The proposals are positive in theory but lack detail. Any guidelines for implementation should be developed in partnership with industry and other stakeholders to ensure they are workable and not unduly complex." (Pfizer)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Clear guidance and an expedited evaluation process were raised by these groups.

"The current codependent evaluation framework is effective for 'simple' pharmacogenetic technologies. No submissions had concerns regarding the methods, only the process delays associated with evaluating these technologies." (Adelaide Health Technology Assessment)

"By providing clear guidance on how evidence should be compiled and considered, these guidelines will enhance consistency and transparency in decision-making regarding pharmacogenomic technologies." (Society of Hospital Pharmacists of Australia)

3.3. Economic evaluation

Topic 3.3. - Overall summary

There was quite a robust discussion in the submissions about economic evaluation and the proposals outlined in Option 3.3. There was broad discussion that economic evaluation should not be used as price negotiation, and many had thoughts and strong opinions on the discount rates and the lowest cost comparator. There were also discussions amongst stakeholders of a desire for broader economic evaluation encompassing additional factors such as environmental impact, ethical, wellbeing and societal benefit elements.

Table 50. 3.3. Economic evaluation: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	33%	50%	0%	17%	12
Pharmaceutical / Medical technology company	0%	5%	21%	74%	0%	19
University or research sector	0%	0%	67%	33%	0%	3
Industry association / Peak body	0%	43%	43%	14%	0%	7
Clinician (or representative organisation)	0%	0%	0%	50%	50%	2
Consulting	0%	33%	67%	0%	0%	3
State / Territory government	-	-	-	-	-	0
Other	0%	25%	50%	25%	0%	4

Patient and Consumer Representative Groups

There was support from these groups for the economic evaluation reforms suggestion in 3.3. Many of these groups did comment that they believed the reforms could take into account broader economic impacts such as patient wellbeing and societal impact. The issue of the current practice of economic evaluation seemingly being treated as price negotiation was also raised.

"In this section, the Options paper makes use of the term "welfare gain", that is the welfare gain to society, to describe where the gain to society from funding a health technology is greater than the cost. The welfare loss should also be considered, where the time taken to negotiate and make decisions, including the need for resubmissions leads to delays in patient access to treatments." (Neurological Alliance Australia)

"The Brain Foundation echoes concerns raised by other stakeholders that the economic evaluation should not be used as price negotiation – as it currently serves in practice. During the evaluation process, parameters are often adjusted to reflect conservative estimates, resulting in a reduced economically justifiable price for new medicines. As a result, sponsors are incentivised to submit a higher initial price, anticipating negotiation and multiple resubmissions. This can lead to prolonged timelines for PBS listing, limiting patient access to essential medicines and increasing costs for both sponsors and the government." (Brain Foundation)

"Economic evaluation should take into account the broader economic impacts of improved health function and quality of life for patients who may be able to make a greater economic and community contribution as a result of more effective medical treatment and improved health outcomes. For example, having access to medicines that more effectively treat Systemic Lupus Erythematosus (SLE) would reduce my burden on the health system through fewer doctors' visits and hospital stays. It would increase my ability to work more hours, enhance my performance at work, and reduce the amount of sick leave I need to take (and given that Australia has a national skills shortage, supporting people to utilise professional skills to their full potential will help the Australian economy). By slowing or preventing organ damage caused by SLE my economic participation would also be extended over my lifetime. As well as improvements in my own quality of life and health outcomes, the burden of care would be reduced for my partner, which would in turn increase his economic participation." (Anonymous submission)

Pharmaceutical / Medical Technology Companies

These companies provided broad support for economic evaluation reforms, but also discussed a great deal on how they believed societal benefits/impacts of new health technologies should be considered, as well as comparators, discount rates and general comments about the valuing of treatments.

"AZ believe broader societal impacts of health technology such as reduced burden for family members or caregivers or better educational outcomes - should be included in economic base cases. The omission of second order effects, social benefits, broader impacts of disability and carer cost and benefit considerations undervalues the societal value of health innovation. Failure to include broader impacts of health innovation leads to undervaluation of health innovation benefits The AZ Consultation 1 Submission focussed on the issue that reimbursement is complex when medicines are supplied by different sponsors, are under patent protection and used in more than one indication. Sponsors may wish to negotiate the prices of component medicines within the combination treatment; however, this is prohibited under the Australian Competition and Consumer Act. Combination therapies are becoming more prevalent. No option to address this issue was presented in the Options paper and failure to identify solutions will impede speed to reimbursement." (AstraZeneca)

"For access to innovative and life changing therapies for Australian rare disease patients it is crucial that reforms are made to comparator selection, discount rates and the assessment of the broader value of medicines. The options paper provides no clear process or pathway for reform in relation to the first two of these and Alexion asks that the final report to government include specific recommendations to address these issues and a clearer staged pathway for the incorporation of broader values. Without change in these areas Australian patients will be left with significantly less options and access to new therapies." (Alexion)

"The options within this section are neutral to negative on the valuation of medicines. Low effective prices are one of the core underlying causes of medicine access delays in Australia. The Valuing Overall option is more positive however lacks sufficient detail." (Eli Lilly Australia)
"One area where significant work and alignment is needed immediately relates to the definition and use of the main comparator in PBAC submissions. Currently there is a disconnect between industry and the PBAC with regards to the definition and use of the main comparator. BMSA supports the position put by our industry body, Medicines Australia, that options relating to comparator selection should be significantly strengthened. In particular, to align with global HTA comparator selection, which is to select the therapy most likely to be replaced in practice. One of the key objectives of the HTA Review is to identify features of HTA which may act as current or future barriers to earliest possible access. BMSA would contend that failure to recognise the value of an innovative medicines by comparing them to the lowest cost comparator in HTA is a clear barrier to early access as it acts as a disincentive to bringing these medicines to patients in Australia." (Bristol Myers Squibb Australia)

This company also went on to recognise *"that the current interpretation of Section 101 (3B) of the national Health Act by the PBAC gives rise to this potential access delay. This could be resolved by better defining what alternative therapy means in the National Health Act. Most simply, section 101 (3B) could be amended to better define alternative therapy as the treatment that is most likely to be replaced in clinical practice. We note that Medicines Australia has also proposed this as an option, along with other alternatives."* (Bristol Myers Squibb Australia)

"Menarini believes that the "Valuing Overall" portion of this option addresses some of the issues which the other portions do not which is what has driven the response above (addresses some but not all of the issues)." (A.Menarini Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These groups were not as convinced that the reforms go far enough and explicitly outlined the issues with the discount rate and the narrow valuing of benefits of technology.

"A technology's environmental impact needs to be included in cost-effectiveness and cost-utility analyses. (i.e. not treated as an externality)" (Health Services Research Association)

"It is difficult to rate these measures because they depend on the outcomes of next steps in the processes. There seems to be significant resistance in the paper to the idea of valuing more broadly the benefits technology brings which is disappointing. Australia should not have a discount rate at the top end of countries in the world." (Medical Technology Association of Australia)

Table 51. Selection of the comparator – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	27%	18%	18%	36%	11
Pharmaceutical / Medical technology company	42%	32%	16%	5%	0%	5%	19
University or research sector	0%	0%	100%	0%	0%	0%	2
Industry association / Peak body	0%	29%	14%	43%	0%	14%	7
Clinician (or representative organisation)	0%	50%	50%	0%	0%	0%	2
Consulting	50%	0%	50%	0%	0%	0%	2
State / Territory government	-	-	-	-	-	-	0
Other	0%	25%	50%	25%	0%	0%	4

The selection of the comparator was widely discussed across all stakeholder groups, many of the Pharmaceutical / Medical Technology Companies believed the current lower cost comparator model stops or delays treatments coming to Australia. There was also commentary from patient and consumer groups, as well as peak bodies about the hindrance of the current lowest price comparator model. A number of stakeholder groups across the board agreed with Medicines Australia's approach on this which is outlined below.

Patient and Consumer Representative Groups

Many of these groups made comments on the selection of the comparator, the discussion ranged from concerns about the lowest cost comparator not reflecting the true value of the treatment, to the opportunities for the comparator to be derived from a consensus generated from RWD.

"The current lowest price comparator model and subsequent price reductions is resulting in newer therapies, that may be first in class, not being brought to Australia, even when given a positive recommendation by the PBAC." (Australian Patient Advocacy Alliance)

"In a market where the comparator is off patent with e.g. bio similar having driven down prices, it will become impossible for new agents to reach patients with IBD. This will have significant consequences for patients who have failed treatment with currently available agents and put them at a distinct disadvantage to patients in other countries." (Monash Children's Hospital)

"In some areas, particularly rare conditions and those health conditions that may require urgency with intervention, there can be variations across the jurisdictions and variations between health services on an accepted standard of care. This can make it challenging for sponsor to identify the comparator without having bias or relying solely on experiences of larger health services. It is important that the opportunity to comment on the comparator is tested broadly and amongst those health care professionals involved in the treatment and care of the rarer or more urgent treatment conditions. Opportunities for the comparator to derive from a consensus generated

from RWD would be helpful, with the caveat that the RWD is quality registry of large enough sample size appropriately representing national case management i.e. again not single centre case registry." (Australasian Leukaemia and Lymphoma Group)

"CCA support the development of guidelines to distinguish between the selection of comparator for submissions claiming superiority and to submissions claiming non-inferiority to make clear which comparator should be selected when there are multiple potential comparators. These groups should include patients who have failed prior therapies that may be being used as the comparator and hence are not clinical options and invalid. Comparisons need to be fair, transparent and realistic. There is also support for listing multiple non-inferior drugs with price equity to minimise supply chain risk that has plagued some medications in IBD over recent years." (Crohn's And Colitis Australia)

"Some members of the NAA expressed concern about the lowest cost comparator policy and its implications for treatment access to the consumer. As quoted in the HTA Options Paper (from Medicines Australia): The lowest cost comparator policy does not reflect the true value of the new therapy because it does not allow pricing at parity to the most commonly used alternative. It acts as a barrier to accessing innovative treatments, which can compound over time as new therapies are also directly or indirectly price-referenced to an older, increasingly rarely used lowest-cost comparator." (Neurological Alliance Australia)

"In HTA economic evaluation, comparators should not be limited to clinical outcomes only, but also consider broader social and lifestyle outcomes such as quality of life, return to work, and a reduction in workload for carers." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

The pharmaceutical companies were very negative about this option and focused on the low-cost comparator and their concerns that it was a barrier and blocker for treatments to come to Australia. They believed that the selection of the comparator should be based upon a weighted approach with the principle of "the therapy most likely to be replaced in practice". There was significant discussion about this option.

"The topic of the lowest cost comparator has been a longstanding concern within the industry. The current legislation was not intended to select the lowest cost comparator. However, due to certain undefined terms (such as cost, over time, the interpretation of section 1013b has tended to favour the lower cost comparator. Boehringer Ingelheim suggests that the HTA review should include an examination of the historical guidelines and recommend greater flexibility in the interpretation of section 1013b to the PBAC." (Boehringer Ingelheim)

"This option requires further consideration and significant change. The selection of comparator should be based upon the principle of "the therapy most likely to be replaced in practice" regardless of the clinical claim being made. The current option proposed does not change current approach to comparator selection or address concerns with use of the lowest cost comparator (LCC). Flow-on pricing impacts through reference pricing due to the PBAC's current application of Section 101(3B) of the National Health Act to cost-minimization submission, has the consequence of devaluing F1 through price erosion over time. Price erosion of F1 medicines risks access to future innovation in Australia. Bayer supports Medicines Australia's recommendation for legislative change to amend the National Health Act to address issues with comparator selection." (Bayer)

"This is a big negative signal to global pharma. The choice of the lowest cost comparator is a blunt instrument that stops medicines coming to Australia or delays their listing on the PBS." (Amgen)

"This option needs to include legislative change to the NHA definition of alternative therapies, otherwise it will not resolve the issue of comparator selection. The Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee outline that the main comparator should be the therapy that prescribers would most replace with the proposed medicine. However, the PBAC has frequently applied a lowest cost comparator approach based on its interpretation of the National Health Act (NHA), Section 101(3B). The Reference Committee also identified concerns about choice of comparator, and the different pricing-related implications and consequences that feed into the PBACs HTA recommendation to the Government. To address these concerns, MSD recommends legislative changes to the NHA definition of alternative therapies to incorporate the PBAC Guideline definition and aligned with the intent of the Strategic Agreement. The lowest cost comparator approach means that PBAC decision making is not always based on the most clinically relevant comparator. This has the impact of undervaluing new innovations that should be compared to the therapy most likely to be replaced. Over time, this can erode pricing for entire classes of medicines or therapeutic areas leading to the Australian standard of care falling behind other similar nations and further disincentivise manufacturers from listing new medicines on the PBS, such as the recent example with Eli Lilly's OMVOH." (MSD Australia)

"For cost-min submissions, the comparator should be what is most likely to be replaced in practice rather than the lowest cost-comparator which in some instances may not be the treatment of choice anymore." (Alexion)

"Whilst careful thought should be given on the operation of section 101(3B) of the National Health Act (1953) the continued ambiguity on the issue of comparator selection in the Consultation Paper is disappointing. The issue of lowest cost comparator is longstanding and a significant barrier to access that worsens with the listing of each new product in the therapeutic area. Novartis, clinicians, other Sponsors in Australia, and Medicines Australia all agree that the appropriate comparator to new a medicine should be the medicine most likely to be replaced in practice, or an appropriately weighted basket of therapies." (Novartis Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Many of the stakeholder groups are aligned with Medicines Australia's position that maintaining the status quo will ultimately disincentivise sponsors from launching early (or at all) in Australia, leading to longer access times, and less choice for patients and clinicians.

Table 52. Valuing of long-term benefits – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	10%	40%	20%	30%	10
Pharmaceutical / Medical technology company	39%	28%	11%	11%	6%	6%	18
University or research sector	0%	0%	100%	0%	0%	0%	2
Industry association / Peak body	0%	17%	0%	67%	0%	17%	6
Clinician (or representative organisation)	0%	0%	50%	50%	0%	0%	2
Consulting	0%	0%	100%	0%	0%	0%	3
State / Territory government	-	-	-	-	-	-	0
Other	0%	0%	33%	33%	33%	0%	3

Patient and Consumer Representative Groups

There was support from these groups, as well as a component of groups that did not know what the impacts of long-term benefits might have had on their organisations. For those who commented on this, they believed that cost-effectiveness was one of the greatest challenges for our country moving forward and if it was not managed it would lead to consumers missing out on the benefits of advances in technology.

"CCA is concerned that the current lowest price comparator model and subsequent price reductions is resulting in newer therapies that can even be first in class not being brought to Australia even when given a positive recommendation by the PBAC. Adapting the comparator and criteria for demonstrating superiority needs to be changed to reflect the actual benefit to the patients - including those with not currently available effective therapy due to prior loss of response or intolerance to all other available classes. Australia risks being seen internationally as an unattractive place to bring new therapies or even involve in clinical trials of new therapies due to the increasingly unlikely possibility of a price that represents actual cost of development and manufacture. This is counter to the goal of increasing access for Australians to best therapies." (Crohn's and Colitis Australia)

"Cost-effectiveness comparisons in the era of rapidly emerging evidence globally will be an ongoing national challenge. The horizon scanning piece would ideally risk-mitigate the decisions made today and the suggested recommendations for re-review." (Australasian Leukaemia and Lymphoma Group)

"Painaustralia supports the valuing of long-term benefits for interventions. Painaustralia reiterates its view that HTA assessment processes must consider the complexity of pain. To effectively do this, cost assessments must adopt societal cost based perspectives that include evaluation of: (i) direct costs and outcomes including direct costs borne by the health care system (for example, drug costs, costs of hospitalisation) and direct outcomes (quality of life impact) on the patient; and (ii) indirect costs, outcomes and effects including productivity loss of patients due

to illness and gains due to participation in the workforce due treatment interventions; and indirect outcomes (quality of life impact) on those affected by caring for an ill patient (for example, carers, parents). Formal HTA processes must also fully value preventative interventions in assessment processes. Assessment processes in addition to reviewing the costs and benefits to the patient and health system must also consider broader societal impacts (including productivity and socio-economic considerations). When the impact of interventions outside the scope of the health system are not factored into assessments the full value of preventative interventions remains unaccounted and those consumers who the community expects to receive the benefit of advances in technology and treatments will actually miss out." (Painaustralia)

"This is essential for rare disease therapies, in particular those therapies like cell and gene therapies that have the potential to be transformative for people living with a rare disease (including family and carers) but have a very high up- front cost. Cost effectiveness guidelines have presented challenges for rare diseases therapies that do not meet LSDP guidelines yet address HUCN." (Rare Voices Australia)

"The value of what price for a life is the essential determinant for all valuing and reviews." (NeuroEndocrine Cancer Australia)

Pharmaceutical / Medical Technology Companies

The overwhelming majority of Pharmaceutical / Medical Technology Companies stressed the need for reductions to the discount rate if Australia was going to keep pace with comparable international peers. They also believed that the current discount rate was under-valuing the long-term benefits of treatments.

"The proposed option to further consider a reduction of the discount rate and conduct more modelling does not move beyond the status quo. Discount rates have already undergone independent assessment, and the PBAC has issued recommendations on this matter. As outlined in the Strategic Agreement (Clause 5.2), Medicines Australia submitted a proposal to the PBAC in January 2022, advocating for a reduction of the base case discount rate from 5% to 1.5%. This adjustment would align Australia with other countries that have lowered their discount rates, recognising the growing importance of long-term health outcomes for their populations. Boehringer Ingelheim recommends maintaining alignment with Medicines Australia, given the substantial effort already invested in examining discount rates." (Boehringer Ingelheim)

"A discount rate of 5% is out of whack with our international peer countries. Out of the 12 countries the Reference Committee referred to that perform HTA that have a 5% discount rate are South Korea and Taiwan. South Korea and Taiwan choose their discount rate based on Australia's and they shouldn't be referenced because of this." (Amgen)

"This option needs to be significantly strengthened with an immediate reduction in the discount rate in line with comparable jurisdictions, otherwise it will not improve value recognition and will undermine the potentially positive outcomes of other options." (Eli Lilly Australia)

"We strongly believe the base case discount rate must be reduced to 1.5%. The current base case discount rate of 5%, has been in place since 1990 and is the highest of 40 countries with established HTA methods. Using 5% as the base case rate means that Australia systematically undervalues vaccines, medicines and other novel treatments that have up-front costs and/or longer-term health benefits. In practice, the use of a higher discount rate means that Australians

face delayed access to a range of vaccines and treatments and in some cases, reimbursed access does not occur at all. A discount rate in line with international best practice would be an important and overdue reform. The proposal (option 3.4) is too marginal and does not address the problem. The option defers the decision resulting in further delay with no timeline for implementation. This is in stark contrast to the Strategic Agreement commitment (Clause 5.2.1) to reduce the discount rate by 2022. The decision to reduce the discount rate should be made and commence by July 2024." (Pfizer)

"We are opposed to keeping the current discount rate for the base-case. Currently, Australia is one of the countries with the highest discounting rate for health economic evaluations which means that heavily undervalues long-term benefits and foregone the benefit of products that have long term benefits. We suggest the reduction of the discount rate to be in line with other comparable jurisdictions." (UCB)

"Australia's discount rate must be lowered in line with international best practice to recognise the value of preventative treatments and cures, and to speed up access. Menarini along with Medicines Australia will advocate for a reduction of the discount rate to 2.5%. If it is left unchanged, it will risk significantly reducing patient access to cutting edge therapies and affecting the long-term future health of generations of Australians, particularly young people who stand to benefit the most from preventative medicines early in their lives." (A.Menarini Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Again, the issue of the discount rate was raised by these groups.

"By developing modelling to assess the aggregate impact of potential changes to the discount rate, decision-makers can better understand the implications on budgetary considerations and opportunity costs. However, careful consideration is needed to ensure that any adjustments to the discount rate align with broader policy goals and do not inadvertently affect the affordability or accessibility of healthcare interventions." (Society of Hospital Pharmacists Australia)

"High discount rates put an emphasis on immediate cost-effectiveness, which undervalues interventions with substantial future health benefits, particularly those addressing chronic conditions. This is especially concerning as high discount rates devalue health technologies for children with chronic conditions by discounting their long-term economic worth. Changes are necessary to ensure equitable access to health technologies and to accurately capture the full spectrum of health outcomes and societal value over time." (Mito Foundation)

Table 53. Valuing overall – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	18%	45%	9%	27%	11
Pharmaceutical / Medical technology company	11%	5%	37%	37%	0%	11%	19
University or research sector	0%	0%	100%	0%	0%	0%	2
Industry association / Peak body	0%	0%	0%	86%	0%	14%	7
Clinician (or representative organisation)	0%	0%	50%	50%	0%	0%	2
Consulting	0%	0%	50%	50%	0%	0%	2
State / Territory government	-	-	-	-	-	-	0
Other	0%	0%	33%	67%	0%	0%	3

Patient and Consumer Representative Groups

These stakeholder groups have provided a great deal of commentary on valuing overall and the ongoing challenges associated with this. One group emphasised that economic evaluation should not be used as price negotiation and there was broad support from a number of groups for the workshops suggested as part of this option.

"It is recognized that this is constant challenge of how to articulate the rational and decisions in lay summary form. Timely information delivered in plain English statements may assist. Consumers will bring lived experience which can assist with the decision making." (Australasian Leukaemia and Lymphoma Group)

"CHF supports the running of workshops that will provide the HTA committees with an understanding of when to accept higher prices for health technologies. CHF agrees that the consultation should include a sample representative of the population, ensuring that there is an adequate number of consumers, and that potential conflicts of interest are disclosed ahead of workshops." (Consumers Health Forum of Australia)

"In addition, concerns have been raised by stakeholders (noted in the HTA Options paper) that economic evaluation should not be used as price negotiation - as it currently serves in practice. During the evaluation process, parameters are often adjusted to reflect conservative estimates, resulting in a reduced economically justifiable price for new medicines. As a result, sponsors are incentivised to submit a higher initial price, anticipating negotiation and multiple resubmissions. This can lead to prolonged timelines for PBS listing, limiting patient access to essential medicines and increasing costs for both sponsors and the government. A focus on the value assessment of new medicines when evaluating new innovative health technologies rather than the undercurrent of cost-minimisation and pricing would address this." (Neurological Alliance Australia)*"As per our comment on the development of an explicit values framework, valuing other aspects of a technology including family and carer impacts, value of knowing and hope and severity of disease combined with HUCN could lead to more equitable outcomes for people living*

with a rare disease assessing cost effectiveness in the context the severe impact and limited options for many people living with a rare disease." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

There was again a large number of very negative comments from this group in regard to valuing overall. However, they did provide extensive commentary for consideration and noted their support for workshops and engagement with the community to understand and quantify the overall value Australians place on health and access to treatments.

"The disregard of societal benefits is a short-sighted approach that is not in line with a person-cantered approach to HTA or to a sustainable healthcare system." (UCB Australia)

"Roche supports engagement with the community to understand and quantify the overall value Australians place on health and access to medicines. However, success of this option will rely on translating the captured sentiment into implementable strategies. Roche recommends the proposed workshops include broader societal value associated with medicine access to reflect the holistic benefit medicines have on Australians and society more broadly. As noted in the options, investment in medicines produces a net welfare gain to society through broader benefits beyond the direct health outcomes, of which little is captured or reflected consistently in current evaluation methods." (Roche Products)

This company also highlighted that *"restricting the inclusion of broader benefits medicines provide on the basis of net welfare gain and producer cost basis models doesn't reflect broader Government evaluation models used in other portfolio areas, such as education (Deloitte 2016). Further clarity is needed to further outline how a broader societal perspective in HTA such that the Budget Operational Rules can support the stated objective of improving Australians wellbeing. Roche requests consultation in proposed workshops on ways to regularly include societal benefit views in HTA evaluations to increase balanced sharing between sponsors and Government of the welfare gain to society from access to medicines. Roche notes that surplus sharing is not a new concept in the PBAC Guidelines (Appendix 6, PBAC Guidelines)*." (Roche Products)

This company also highlighted that "restricting the inclusion of broader benefits medicines provide on the basis of net welfare gain and producer cost basis models doesn't reflect broader Government evaluation models used in other portfolio areas, such as education (Deloitte 2016). Further clarity is needed to further outline how a broader societal perspective in HTA such that the Budget Operational Rules can support the stated objective of improving Australians wellbeing. Roche requests consultation in proposed workshops on ways to regularly include societal benefit views in HTA evaluations to increase balanced sharing between sponsors and Government of the welfare gain to society from access to medicines. Roche notes that surplus sharing is not a new concept in the PBAC Guidelines (Appendix 6, PBAC Guidelines)." (Roche Products)

"AZ welcomes the HTA Review option paper suggestion of conducting workshops to understand if and where it may be reasonable for HTA committees to accept higher prices for health technologies. The AZ Consultation 1 Submission highlighted that PBAC usually manages uncertainty by using highly conservative estimates rather than most likely estimates in economic evaluation and estimation of utilisation and budget impact. This includes time horizons of

economic evaluations being truncated, convergence of effectiveness and unreasonable parameters in sensitivity analyses to assess the degree of uncertainty. This approach leads to low values for innovative health products and consequent prices for medicines seeking reimbursement. The low value assigned to medicines is a fundamental issue constraining the speed of patient access to medicine in Australia." (AstraZeneca)This company also expanded by saying that *"Without an effective framework to manage risk, it is challenging for the PBAC to subjectively assess and balance risks of opportunity cost. As such, AZ supports the development of a framework and policies for identifying the risks associated with key uncertainties and formulation of associated risk management plans (RMP) that manage these risks. Risk management planning should articulate parameters such as cost-effective populations, market increasing potential for the new product, impacts of a new entrant, and accommodate renegotiation of expenditure caps."* (AstraZeneca)

"Alexion supports the development of a broader value framework incorporated into the economic analysis so long as the flexibility in the appraisal process remains. Broader value should flow into the base case of the economic models, the care giver benefits and where the therapeutic benefit improves functional benefit/morbidity. Explicit commitment should be given to develop and include second order benefits in the value assessment where appropriate." (Alexion)

"Ensuring the value of innovation and true sharing of clinical, economic and financial uncertainty are recognised as key elements that are required within any new pathways defined to reduce the timeline between TGA registration and PBS listing. These factors need to incorporated within pathway recommendations to the Minister of Health and Ageing." (Bristol Myers Squibb Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Many of these groups were neutral or positive in regard to reforming the valuing process overall. They made a few comments about the importance of stakeholder engagement for including a broad range of perspectives and expanding the factors contributing to benefits.

"By engaging stakeholders and considering various factors such as the magnitude of benefit, confidence in achieving that benefit, and potential measures to offset higher costs, HTA committees can make more informed decisions. Ensuring representation from a diverse range of stakeholders and utilising a qualitative value framework can help capture a broad spectrum of perspectives and mitigate biases in decision-making." (Society of Hospital Pharmacists Australia)

"How do you value a $400 vaccine that last 12 years or a drug $40 a month that needs to be taken every day by consumer" (Immunisation Coalition)

"Traditional HTA models often focus on clinical and economic outcomes, such as mortality, morbidity, and cost-effectiveness. However, recognizing and incorporating broader impacts, including psychosocial benefits, is increasingly recognized as important for a comprehensive evaluation, and should be considered in this. We have provided examples of other factors that should be considered in this process in the explicit qualitative value framework section." (Mito Foundation)

"We would like to reemphasise the importance of price negotiations at multiple points in the HTA system - price policy should be more much explicitly addressed in the Options Paper in relation to listing for Aboriginal and Torres Strait Islander health technologies on MBS and PBS. Specifically, the payer should have structured means to pay a higher price for an item that will disproportionately benefit Aboriginal and Torres Strait Islander people. Such an approach is a practical way in meeting equity needs expressed in the National Medicines Policy" (NACCHO).

"A focus on cost minimisation, fixed discounting and disinvestment and selection of the cheapest comparator rather than investment and ascertainment of societal benefits would appear to be a negative and may dissuade investment by global companies in the Australian market...Nobody wins with that outcome... as we see in New Zealand. We do not want anything like a Pharmac model here." (Clinician)

Section 4: Health Technology funding and purchasing mechanisms and decisions

Stakeholders were invited to provide written comment on the reform options presented for Health Technology funding and purchasing mechanisms and decisions as per the table below (reproduced from the HTA Review's Options Paper).

Subject	Key option/s
4. Health technology funding and purchasing approaches and managing uncertainty	
4.1. Approaches to funding or purchasing new health technologies	
Recognising competition between new health technologies that deliver similar outcomes	Alternative option 1: In conjunction with options for proportionate assessment of cost-minimisation submissions (see Proportionate appraisal pathways), require offers of a lower price for health technologies that provide no added benefit. New therapies that offer no advantage in terms of improved efficacy or safety (i.e. no improved health outcomes), would be required to offer a lower price to be funded. Further work will need to be done to determine the parameters around the cost-minimisation submissions this would apply including defining the circumstances where it would be appropriate to apply these policies. **OR** Alternative option 2: In conjunction with options for proportionate assessment of cost-minimisation submissions (see Proportionate appraisal pathways), incentivise offers of a lower price for health technologies that provide no added benefit. New therapies that offer no advantage in terms of improved efficacy or safety (i.e. no improved health outcomes), would be encouraged to offer a lower price to be funded. Further work will need to be done to determine the parameters around the cost-minimisation submissions this would apply including defining the circumstances where it would be appropriate to apply these policies and quid pro quo options.

Investigate further options to address budget impact implications of high-cost/high impact health technologies	Identify appropriate alternate contract funding/financing tools and instruments (e.g. annuity payments, patient-level product warranties) in consultation with stakeholders to address budget implications of high-cost/high-impact health technologies in the Australian context. This work should focus on instruments that may help to address: 1. clinical, financial or economic uncertainty (see Approaches for managing uncertainty) 2. resolving issues in submissions that prevent positive recommendations being made (see early resolution options) 3. addressing lack of incentive for developing health technologies in certain areas (see Approaches to incentivise development of products that address antimicrobial resistance)
Pricing offer (PO) and negotiation guidance framework	Introduction of a PO and negotiation guidance framework for health technologies that have been approved by the TGA and positively recommended by a HTA Committee, which accounts for the comparative/incremental health benefit of the health technologies compared to existing available subsidised products, as well as overall budget impact implications. Such prescriptive frameworks exist in a number of European healthcare systems where HTA evaluations explicitly influence reimbursement/pricing negotiation parameters. This framework may be designed to apply to: a. all health technologies submitted for HTA evaluation; b. health technologies submitted for HTA evaluation on a cost-minimisation basis; or c. specific health technologies that meet defined criteria (e.g. advanced therapies, first-in-class therapies of high clinical benefit that address unmet need, health technologies that support measures to address health equity and/or other priority areas)
Post-listing re-assessment of health technologies	Introduction of a systematic and enhanced, rapid program that (re-) reviews health technologies to provide funding/purchasing and disinvestment advice to the HTA Committee for consideration at set periodic intervals after the initial HTA evaluation. As part of establishing this standing program, an explicit disinvestment framework should also be designed and communicated to stakeholders after appropriate consultations.

Approaches for managing uncertainty - bridging funding coverage for earlier access to therapies of likely HATV and HUCN	Establish bridging funding through a capped special funding program (separate and distinct from the PBS special appropriations) or legislate to enable conditional listings on the PBS. The purpose of either of these options would be to provide for a time-limited period, bridging funding coverage for earlier access to exceptionally promising, time-critical, therapies of HATV and HUCN, but that have significant clinical, economic and/or financial-based uncertainty. The program would need to be designed in a way that does not introduce further complexity into the system nor create perverse incentives that would prolong assessment and commercial negotiations. The design of this program should incorporate specific details on the eligibility requirements that health technologies need to meet to qualify for funding from this program that aligned with the core HTA and pricing negotiation steps that are features of the Australian HTA process and include, but not be limited to: 1. Early identification and nomination via horizon scanning and/or designation on a Priority List of HUCN conditions. 2. Eligibility requirements to lodge TGA and PBAC submissions (simultaneously) for the health technology within 6 months of receiving first international regulatory approval (i.e. FDA/EMA) 3. Requirement for parallel TGA/HTA Committee submission lodgement as part of a broader overall approach to support timely recommendations. 4. Approach undertaken by the applications and evaluation that: o provides the HTA committee with options to make recommendations for interim conditions of funding for the purposes of bridging access, and recommendations that inform further price and access negotiations; or o facilitates finalisation of price and access negotiations between the sponsor and the healthcare payer prior to presentation to the HTA committee for consideration. • Administration that enables clinical data to be collected and reviewed. • A clear process for re-assessment and final decision-making on whether (and when) the health technology should transition onto ongoing funding arrangements (such as the PBS, MBS or NHRA-style arrangements, with or without additional evidence development), or whether bridging funding should be withdrawn. Such decisions would be based on what pre-defined evidence has accrued during the time limited period, and whether the health technology is performing as anticipated.
Approaches for managing uncertainty - revised guidance on the	Revised guidance and policy arrangements that encourage the creative proposition and utilisation of managed entry arrangement instruments by the respective parties, supported by more explicit HTA committee recommendations enabled by appropriate changes to current policy and legislation, would facilitate greater uptake and provide more options to sponsors and the Commonwealth to

uses of different managed entry tools	engage with uncertainty more constructively and collaboratively, as part of improving timely access to health technologies. Note: this may need to be accompanied by changes to negotiation guidance, policy, regulations and/or legislation to facilitate implementation.

4.2. Approaches to incentivise development of products that address antimicrobial resistance (AMR)

HTA Fee exemptions for products that address AMR	Explicitly include antimicrobial health technologies that address the public health risks associated with organisms on the WHO bacterial/fungal priority pathogen lists as HTA fee exempt in regulations would be appropriate as part of a broader set of incentives and reforms.
HTA Policy and Guidance changes for products that address AMR	The Department of Health and Aged Care has commenced work towards identifying and scoping potential funding mechanisms and economic models to incentivise market availability of antimicrobial products in Australia, the Reference Committee Use this work program to examine how targeted changes to HTA policy and methods regarding PICO definitions, evaluation of clinical evidence and dimensions of value for antimicrobial products (e.g. by drawing on the experience of the National Institute for Health and Care Excellence (NICE)/National Health Service (NHS) pilot and the application of the "Spectrum, Transmission, Enablement, Diversity, Insurance Value (STEDI)" value framework) could be applied in practice, given the public health significance and implications of AMR. Workshop variations to the standard HTA evaluation approach for health technologies that should be evaluated further as part of a prospective work program.
Funding and reimbursement-related changes to support availability of antimicrobials	Workshop a possible option that recommends the Government examine and test multiple payment and incentive models (including, but not limited to full and partial price/volume delinking, advance market commitments, guarantee-of-supply provisions) as part of designing a flexible reimbursement policy in respect of antimicrobial products purchasing.

Oversight – reforms to optimise access to and use of RWD in HTA	Establish a multi-stakeholder advisory group, reporting to government, to co-design and oversee the development and implementation of enabling systems, pathways, evaluation, and research to optimise access and use of RWD in HTA.
Develop a strategic approach to increase confidence, awareness, and acceptance of cross-jurisdictional and cross-sectoral RWD access and use in HTA	This approach should centre consumer and community engagement and co-design, leverage and integrate existing international activities and guidelines, incorporate Australian context and evidence, and fine tune responses and messages specific to HTA. Critically, Australia should continue to develop and enhance systems that ensure privacy protections and data security. Australia could develop a strategic approach to increase confidence, awareness, and acceptance of cross-jurisdictional and cross-sectoral RWD access and use in HTA. This approach should centre consumer and community engagement and co-design, leverage and integrate existing international activities and guidelines, incorporate Australian context and evidence, and fine tune responses and messages specific to HTA. Critically, Australia should continue to develop and enhance systems that ensure privacy protections and data security.
Data infrastructure	Develop a dynamic, enduring whole-of-government data infrastructure, including transparent and streamlined governance, that is fit-for-purpose to accelerate RWE development for HTA. 1. This infrastructure should evolve over time, based on the needs of HTA agencies and other stakeholders. 2. It should also be harmonised using international standards, be flexible to accommodate treatment landscape changes, scalable to incorporate emerging novel datasets, and allow transparent data quality assessment. 3. Integrated health and social data from a single populous jurisdiction may be fit-for-purpose to address some research questions. These data may be more rapidly accessible and offer depth across multiple sectors.
Methods development	Develop a multi-stakeholder coordinated approach to transparent evidence development using best-practice methods for HTA, spanning data standardisation, standardised analytics, and reporting.

Develop Guidance framework	Guidance on the use of RWD and RWE would be produced under the oversight of the aforementioned advisory group, following the development of methods. In the interim, the FDA data standardisation framework adopted by the TGA may also be adopted to guide the use of RWD in HTA for subsidy decisions.
Collection of utilisation and outcome data for provisionally listed health technologies	Existing national or international registries should be used, where possible, to facilitate the collection of outcome data relating to provisionally listed technologies in a timely manner. 1. Outcomes of interest should be determined based on the areas of uncertainty to be resolved, along with baseline data and information relating to other care received. 2. When it is expected that an application is likely to result in a CED arrangement, a suitable registry should be identified as early as possible, and negotiations commenced to determine the feasibility of data collection and timely access, as well as resourcing requirements (to be paid for by the sponsor, under cost-recovery arrangements). In the longer-term outcomes of interest may be collected as an add-on to relevant enduring data-linkages or e-Health Record data, as recommended by the advisory body (above). 3. In the case of ultra-rare diseases, international registries should be utilised. Prior to entry into any CED arrangements, the likelihood of obtaining new evidence to address areas of uncertainty should be considered.

Section 4 – Overall summary

Many patient groups were supportive of measures that could potentially make access to health technologies more affordable for their patients and the broader community. It was also recognised that there needs to be a submission pathway open to non-commercial sponsors where there is no commercial imperative for a company but there is critical clinical need for small population groups.

However, both reform options for cost minimisation submissions put forward at 4.1 were rejected unilaterally by pharmaceutical industry stakeholders on concerns such measures will see fewer products brought to market and ultimately limiting patient choice. It was also commonly noted that a narrow focus on clinical efficacy and toxicity fails to recognise benefits such as improved quality of life and/or burden of treatment for patients that should be appropriately considered in the determination of value. It was argued that a matching price would be a more workable solution.

Another issue of contention related to the reform option relating to post-market assessment. While acknowledging a need to continue to review performance of a funded technology in market, many suggested current arrangements to facilitate this are sufficient. There were some concerns identified on the resource impost this could place on the HTA in terms of taking scarce resources from the assessment of new or emerging health technologies.

The concept of bridging funding held strong intuitive appeal for most. Pharmaceutical / Medical Technology Companies noted that more transparent and equitable risk sharing models would need to be developed to ensure the most innovative/uncertain technologies could leverage such a pathway.

The other reform options in this section were broadly supported across stakeholders, albeit with a common view that further consultation and co-design would be required if taken forward.

4.1. Approaches to funding or purchasing new health technologies

Table 54. 4.1. Approaches to funding or purchasing new health technologies: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Sample size
Patient or consumer (or representative organisation)	0%	78%	22%	0%	9
Pharmaceutical / Medical technology company	0%	0%	32%	68%	22
University or research sector	0%	33%	33%	33%	3
Industry association / Peak body	0%	29%	71%	0%	7
Clinician (or representative organisation)	-	-	-	-	0
Consulting	0%	0%	100%	0%	3
State / Territory government	0%	100%	0%	0%	1
Other	0%	20%	60%	20%	5

There was a clear divergence of opinion on this proposed reform across key stakeholder subgroups as highlighted below.

Patients, Consumers and Representative Groups

Some (9) patient groups were supportive of measures that could potentially make access to health technologies more affordable for their patients and the broader community. It was also recognised that there needs to be a submission pathway open to non-commercial sponsors where there is no commercial imperative for a company but there is critical clinical need for small population groups.

"CHF supports HTA reform that counterbalances the monopolistic tendencies of some technologies, and that stimulates downward pressure on prices, and as a result increasing affordable access. Even if this does not necessarily translate into a decrease in out-of-pocket expenditure at the pharmacy (some technology might become cheaper but be above the PBS general patient charge), it still delivers better value for money to consumers through a better use of taxation revenue." (Consumers Health Forum of Australia)

"The NAA supports the proposals in this section that have the potential to improve timely and equitable access for consumers. In addition, there should be a clear process to enable submissions by organisations other than Pharmaceutical / Medical Technology Companies such as peak consumer or clinical bodies or a consortium of these bodies, such as the submission in 2010 to list nicotine patches on the PBS made by a consortium consisting of Cancer Council Australia, Heart Foundation, Australian Council on Smoking and Health and Quit Victoria." (Neurological Alliance Australia)

"The options proposed in this section have the potential to positively impact for people living a rare disease. However, these should also be an option that supports a submission by non- commercial sponsors as well. This is in line with the recommendations in The New Frontier – Delivering better health for all Australians report as well." (Mito Foundation)

"All options in this section to facilitate early access to technologies with HUCN/HATV (Options to address budget impact of high cost/high impact health technologies; Approaches to managing uncertainty) have the potential to have a positive impact on timely and equitable access for people living with a rare disease. A critical gap is an option to support submissions by a non-commercial sponsor such as professional clinical body, peak group or consumer body where there is no commercial incentive for a company e.g. in the case of access to treatment subsidised for a common condition but not a rare condition. The New Frontiers report recommended that a fund be established for this purpose." (Rare Voices Australia)

One stakeholder called for a broader recognition of value/benefits to patients beyond specific clinical endpoints and a need for product choice for consumers to also be considered.

"As discussed in 1.2, we note that this option states 'new therapies that offer no advantage in terms of improved efficacy and safety (i.e., no improved health outcomes) would be required to offer a lower price to be funded' and are concerned at the narrow definition of 'health outcomes' that does not take account of health outcomes and indirect benefits beyond specific clinical endpoints. How will this approach improve the current situation with new therapies if it can only take into account the very limited morbidity measures and not factor in the value for money of other health outcomes and benefits? Moreover, if the new therapy is competing in cost with an existing therapy that has been purchased by government at a discounted price, there is an automatic competitive imbalance in the process." (Haemophilia Foundation Australia)

Pharmaceutical / Medical Technology Companies

While some elements of these reforms were supported (e.g. the bridging funding mechanism), there were strong concerns expressed about an explicit requirement for lower prices for health technologies that deliver similar outcomes. It was argued that such a reform would likely lead to fewer products brought to the Australian market. Other comments focussed on disinvestment related reforms and the need for any new process to be both rigorous and transparent to all stakeholders.

"Whilst there are some positives, there are some don't knows and a clear negative with CMA price reductions - hence address little or none of the issue." (Johnson and Johnson Innovative Medicines)

"The Options paper addresses some issues associated with funding and purchasing mechanisms and decisions. Proposed options associated with alternative instruments and financing tools to address budget impact and time-limited funding for therapies to address areas of high unmet clinical need (HUCN) could help in achieving HTA Review outcomes. Eligibility for the conditional listing pathway needs to be expanded to maximise the benefits of this option. The option that health technologies which deliver similar outcomes should list at lower prices than current technologies on the market would slow time to access and create inequity. Any changes to a post-listing reassessment framework for health technologies needs to involve the same level of analytical rigour of that at listing, stakeholders must be consulted, and results transparently communicated." (AstraZeneca)

"Options to mandate or incentivise lower prices for cost-minimisation submissions will have a major impact on the availability of new therapies for patients in Australia and Alexion strongly opposes both options. Alexion opposes the development of a disinvestment framework, which is unnecessary. Alexion supports the principle of a bridging fund but the options presented need strengthening and refinement." (Alexion)

"As discussed in the streamlined submission section, Novartis Australia does not support any implied speed versus price trade off with the streamlined submission pathway that requires cost-minimisation submissions to offer or accept a lower price because of this pathway being chosen. For technologies which provide no additional benefit of efficacy or safety, a cost-minimisation analysis provides a well-accepted approach to HTA evaluation and is currently used by the PBAC and MSAC and is widely applied overseas. Although the proposal suggests an improvement in the listing timeline by a few months, there is no reason why this acceleration could not be applied in the current context where the same price is offered. To request lower prices would have flow on effects to other sponsors which have listings in the same therapeutic area under the current 'price referencing' policy." (Novartis Australia)

"BMSA believes that the options put forward with regards to recognizing competition between new health technologies that deliver similar outcomes has the potential to not only exacerbate the time to access issues we currently have in Australia, but to also see global pharmaceutical organisations de-prioritise Australia as a first-wave launch country. Global organisations consistently challenge their Australian affiliates with regards to the low net pricing in Australia compared to other developed countries. Pricing certainty within F1 once listed on the PBS is the major contributor to keeping Australia within first-wave launch countries. BMSA sees the options detailed in Chapter 4 as reducing this pricing certainty for both cost-minimisation and cost-effectiveness medicines, and as such risks Australia being de-prioritised with regards to launching new medicines." (Bristol Myers Squibb Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

One State government stakeholder noted that while they were supportive of measures to reduce budget impacts, the value of highly specialised therapies (HST's) needs to reflect the long-term clinical outcomes.

Other stakeholders echoed the concerns of the pharmaceutical sector on the issue of price discounts and a need to ensure our system encourages companies to bring new therapies to the Australian market.

"The approach to require a price discount to list for a cost-minimisation submission is problematic as this in itself is a disincentive to bring medicines to Australia. It appears oversimplified in the option paper that differences in patient response can exist and these warrant alternatives in the market. This also can reduce choice in mid to long term and can have unintended policy impact." (Consultant)

"Major advances like mRNA vaccine, cell-based vaccines and adjuvants need to have a way of being looked at early if is clinically useful." (Immunisation Coalition)

Table 55. Recognising competition between new health technologies that deliver similar outcomes: Alternative option 1: In conjunction with options for proportionate assessment of cost-minimisation submissions, require offers of a lower price for health technologies that provide no added benefit

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	13%	0%	38%	13%	0%	38%	8
Pharmaceutical / Medical technology company	86%	9%	0%	0%	0%	5%	22
University or research sector	0%	0%	67%	33%	0%	0%	3
Industry association / Peak body	50%	17%	0%	17%	0%	17%	6
Clinician (or representative organisation)	0%	0%	0%	0%	0%	100%	1
Consulting	25%	50%	0%	0%	0%	25%	4
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	0%	0%	33%	33%	0%	33%	3

Patients, Consumers and Representative Groups

There was limited additional comment on this specific reform option from patient and consumer groups. Those that did comment noted that valuations of health technologies need to encompass a broader perspective rather than solely focussing on clinical outcomes, and that cost should not be afforded higher priority than clinical need and health equity considerations.

"Competition should not be limited to clinical outcomes only, but also consider broader social/lifestyle outcomes such as delivering improvements in quality of life, earlier return to work. and reductions in workload for carers." (Consumers Health Forum of Australia)

"Several recommendations in this section need further detail and ongoing measurement to ensure that they achieve their desired aim. Whilst budget implications need to be considered, therapies should be considered with an investment paradigm. Cost/benefit and quality of life/community/society impact should be given appropriate consideration. Cost cannot be the overriding factor in access to approved therapies for eligible Australians" (Collaborative Consumer Group Response)

Pharmaceutical / Medical Technology Companies

Most pharmaceutical stakeholders overtly rejected the concept of a price reduction being needed for cost-minimisation submissions. Many argued that such a reform would see less products being made available for Australian consumers – even when provision of the same drug in a different format delivered significant convenience value for the patient. Some also argued that pricing issues were beyond the HTA Review Terms of Reference.

"We appreciate that this pathway can potentially streamline the process of a similar medicine (no advantage in terms of improved efficacy or safety), however there are concerns about the comment of "would be required to offer a lower price to be funded". We cannot use this as a new mechanism for price saving or price reduction measures. In terms of the 'lower price', this is very ambiguous, and we cannot support this statement without fully understanding the unintended consequences. It is reasonable when doing a current cost-minimisation submission to have an equivalent price to the comparator, so this should be our starting point. A lower price, what does this constitute in terms of implementation? A set % reduction or at the discretion of the PBAC committee? The other important detail which we would need to see more of is who/how do we determine improved safety or efficacy? We understand that we must show equivalence in our submissions to the comparator, however there is no mention about patient reported outcomes or benefits to patients in the assessment. For example, we might have a medicine that is an oral tablet and is equivalent in efficacy and safety to an IV drug. However, there could be circumstances where an oral tablet is far more convenient for a patient to take and access - but this is not taken into consideration. If we are striving to put a patient at the centre of this process then we should also include these insights into decision-making." (Antengene Australia)

"We support creation of a proportionate assessment process which will lighten the load on HTA bodies and could accelerate access. However, coupling a proportionate assessment pathway with price reductions is inappropriate and unacceptable. This would result in further devaluation of health technologies and undermine Australia's international competitiveness with further delays to access. This proposal appears to be an attempt to introduce pricing policy into the HTA review when it was not included in the terms of reference. The proposal creates the risk of creating cyclical price reductions because of reference pricing when these therapies are later used as comparators which will further undermine value. Linking price reduction to a measure intended to support faster access cannot be supported and creation of a quid pro quo with faster processing achieved in exchange for cost reductions fails to recognise the fundamental problem that evaluation for low risk/low budget impact products is overly cumbersome and hence too slow. Progressing the proportionate assessment pathway should be based on improving efficiency of the system and improving speed to access." (Pfizer)

"AZ reject the option for Sponsors to offer a lower price for health technologies that provide no added benefit. It will decrease speed to access and lead to inequity. Requiring Sponsors to offer of a lower price for health technologies that provide a similar clinical benefit will decrease therapeutic choices for patients. Adoption of this approach would have broad and far-reaching impacts, as government reference pricing would create downward discounting compounded by anniversary price reductions and F2 price cuts. The Options paper also notes that there may be clinical areas where patient response to treatment is heterogeneous and for clinical reasons having a range of treatment options is necessary for achieving overall optimal outcomes for patients. Reducing the price for products of similar therapeutic value will reduce commercial incentives to seek reimbursement and limit the available range of treatments." (AstraZeneca)

"Such a measure should not appear in the Reference Committee's final report. Price-reduction measures are outside the terms of reference for this Review. Implementation of such an option, risks fewer medicines coming to market and consequently less choice for patients and clinicians. This approach ignores that two medicines with a similar efficacy or safety benefit may have other benefits that justify similar pricing rather than a lower price. The Australian system already has many price controls, statutory price reductions, reference pricing and post-market reviews; it not necessary for this review to introduce new price saving or price reduction measures." (Bayer Pharmaceuticals ANZ)

"We strongly oppose the implementation of a lower price for the lowest cost comparator. The rationale for this proposal seems to ignore that the support of subsidised healthcare in Australia operates completely outside the ranges of a free market system. If two medicines confer similar benefit, there is no HTA-based reason to justify a lower price. Price reduction proposals were outside the defined scope of the Review." (UCB Australia)

"Roche strongly disagrees with this option and believes it will have a detrimental impact on access to new health technologies. A significant number of pricing and cost control mechanisms are already in place on the PBS, and further pricing controls are unwarranted. From a Roche perspective, this option will lead to significant delays to important treatment options for patients, and result in Australia becoming less attractive as a first launch market. A mature and well-administered healthcare system recognises a cost-minimisation analysis is a methodology to assess the impact of a therapy, and not a reflection that a therapy provides no added benefit to patients or the healthcare system; we note this proposed option would infer a newer therapy applying as a cost-min-minus would be the latter, whereas this approach would suggest the new therapy is inferior to the standard of care, which is evidently not the case. If this option was in place today, several Roche therapies which provide important treatment options and benefits for patients and clinicians but assessed on a cost-minimisation basis due to limitations with the current HTA process, would simply not be available in Australia. Australia is already considered a low-price country in the global context and price parity and/or appropriate relativity to other therapies within the same class is a bare minimum requirement to enable these treatments to come to market." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

A number of these stakeholders noted that while price reductions for drugs providing the same safety and clinical outcomes as those in the market make a degree of sense, the potential cost savings of such a measure need to be weighed carefully against both security of supply and innovation considerations.

"While I am supportive of some sort of price penalty for "me-too" drugs, it is important that this is not so harsh that it generates insecurity of supply. Also, some "me-too" drugs have different formulations or allow greater efficiencies in delivery and these are important for health system functioning. So, "New therapies that offer no advantage in terms of improved efficacy or safety (i.e. no improved health outcomes), would be required to offer a lower price to be funded." might need to be extended to account also for the concept of health system efficiency/quality use of medicines." (Adelaide Health Technology Assessment)

"The requirement model may inadvertently deter market entry which may negatively impact other activities such as clinical research that may benefit from this being in market. This may have

unintended consequences given the relatively small size of the Australian market." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

Table 56. Recognising competition between new health technologies that deliver similar outcomes: Alternative option 2: In conjunction with options for proportionate assessment of cost-minimisation submissions, incentivise offers of a lower price for health technologies that provide no added benefit

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	25%	38%	0%	38%	8
Pharmaceutical / Medical technology company	86%	10%	0%	0%	0%	5%	21
University or research sector	0%	0%	67%	33%	0%	0%	3
Industry association / Peak body	17%	33%	17%	17%	0%	17%	6
Clinician (or representative organisation)	0%	0%	0%	0%	0%	100%	1
Consulting	25%	25%	25%	0%	0%	25%	4
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	25%	0%	25%	25%	0%	25%	4

Feedback across stakeholder groups was very consistent with that observed for Alternative 1 above (and as such is not repeated here).

Table 57. Investigate further options to address budget impact implications of high-cost/high impact health technologies – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	13%	0%	25%	50%	13%	8
Pharmaceutical / Medical technology company	16%	5%	26%	26%	0%	26%	19
University or research sector	0%	0%	67%	33%	0%	0%	3
Industry association / Peak body	0%	0%	17%	67%	0%	17%	6
Clinician (or representative organisation)	0%	0%	100%	0%	0%	0%	1
Consulting	0%	0%	0%	67%	0%	33%	3
State / Territory government	0%	0%	0%	100%	0%	0%	2
Other	0%	0%	33%	67%	0%	0%	3

Patients, Consumers and Representative Groups

Patient groups were supportive of this proposed reform given its scope to encourage the provision of higher cost, more specialised treatments that otherwise may not be commercially viable to bring to market.

"We fully support this as a critical option to address rare disease therapies that do not meet LSDP criteria but are high cost/high impact technologies. A gap in the options is incentivising submissions for technologies that are likely to reduce the treatment burden for rare disease patients. For example, many rare disease therapies present a significant burden such as weekly infusions in an outpatient setting, this has significant impact on patient experience and quality of life, cost minimisation measures should not disincentivise the development of alternative delivery mechanism that will reduce the burden of treatment." (Rare Voices Australia)

"Advanced therapeutics for childhood dementia disorders are coming through the pipeline, options need to be available for these treatments that have a high up-front cost but long-term benefits in a population that has high unmet need." (Childhood Dementia Initiative)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies were also broadly supportive of this reform, albeit with a need for additional stakeholder consultation and co-design if this option is to be taken forward for implementation.

"AZ supports research to identify and co-create alternate contract funding and financing tools. This includes research to identify and co-create alternate contract funding and financing tools (e.g., annuity payments, patient-level product warranties) for high-impact health technologies in Australia." (AstraZeneca)

"The implementation of initiatives to manage budget impact is positive. In developing those initiatives, it is important adequate consultation is undertaken with stakeholders including industry." (Pfizer)

"Roche supports investigating further options to identify appropriate alternate funding tools and instruments to address budget impact implications of high-cost/high impact health technologies. Roche would seek further consultation on this option with further detail shared on what tools are being considered and how implementation may be achieved, whilst ensuring that any new funding mechanisms do not result in further delays or establish inequities to patient access." (Roche Products)

Others said they needed additional detail on this specific reform before they were able to indicate if this would be supported – especially in terms of how risk would be shared between government and industry.

"We welcome the investigation of alternative contract/funding/financing tools for HC/HI technologies, however without the details and the criteria for this to be a consideration, we cannot support this at this time." (Antengene Australia)

"The opportunity to contribute to the identification of alternatives is welcomed, but since no options were really proposed it is difficult to comment." (UCB Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholders also provided qualified support for this reform, noting the challenge in getting this right rests in striking the right balance between structure and flexibility (e.g. not necessarily seeking a one size fits all solution).

"I am positive about this because I think the existing cap and rebate risk share arrangements are very blunt tools and don't necessarily achieve the desired outcomes for either party. I understand they have probably evolved like this due to the administrative burden of implementing these RSAs. However, some more creativity and flexibility in the structure of these arrangements should be considered - whilst noting they have to be implementable." (THEMA Consulting)

"Introducing alternate contract funding/financing tools and instruments may add complexity to the funding landscape, requiring stakeholders to navigate unfamiliar mechanisms and processes. This complexity could potentially delay access to new technologies or increase administrative burden for healthcare providers and payers." (Society of Hospital Pharmacists of Australia)

Table 58. Pricing offer (PO) and negotiation guidance framework – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	29%	14%	14%	43%	7
Pharmaceutical / Medical technology company	5%	29%	24%	14%	0%	29%	21
University or research sector	0%	0%	100%	0%	0%	0%	3
Industry association / Peak body	0%	0%	17%	50%	0%	33%	6
Clinician (or representative organisation)	0%	0%	0%	0%	0%	100%	1
Consulting	0%	0%	0%	67%	0%	33%	3
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	0%	33%	67%	0%	0%	3

Patients, Consumers and Representative Groups

Some patient and consumer groups were supportive of this reform as a means of potentially reducing time delays observed between positive funding recommendations and consumer/patient access.

"CHF supports the introduction of a pricing offer and negotiation guidance framework. As discussed above, incremental health benefits must consider broader social/lifestyle outcomes of the health technologies." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

Some Pharmaceutical / Medical Technology Companies offered a degree of qualified support for this reform, but many suggested this would need to be co-designed with stakeholders to ensure it does deliver more timely access and does not potentially induce further delays.

"Any implementation of a PO framework needs to ensure that the timing of listing from recommendation is faster than currently and not lead to further delays." (Alexion)

"Such a framework should only be implemented if it will speed access and not create additional steps that slow this down. If this framework is to proceed it should be aligned with cost-effectiveness principles and co-designed with industry stakeholders." (Bayer Pharmaceuticals ANZ)

"The HTA Committee noted that the amount of time required after a PBAC recommendation to negotiate pricing and PBS listing arrangements is inefficient. A post-PBAC pricing and listing process framework with target timeframes for commencing and finalising each step is required. Improved transparency of progress through the process and engagement between Sponsors and the DoHA is also needed. Independence of the HTA evaluation through separation between the PBAC's consideration of cost-effectiveness from the Government's consideration of budget impact and expenditure cap negotiations will improve efficiency, reduce the number of resubmissions and reduce the time that patients wait for PBS access to new medicines. PBAC recommendations regarding assumptions of utilisation should be limited to advice on the relationship between utilisation and cost-effectiveness, rather than estimates of uptake among cost-effective populations. Also, subsequent adjustments to the financial estimates that do not relate to pricing and budget impact negotiations are a significant cause of resubmission and PBS listing delay. The framework should reflect this requirement for independence in the process of cost-effectiveness evaluation." (AstraZeneca)

Other companies did not support this reform, suggesting it may cause greater delays.

"Novartis Australia is unsure as to how this step in the process would meet the objectives of the HTA review. Fundamentally the PBAC should set the value and the subsequent price of the medicine in question. It is not appropriate for there to be a further negotiation following this, which accounts for the comparative/incremental health benefit of the health technologies compared to existing available subsidised products, as well as overall budget impact implications. This would only further delay access to medicines and detrimentally impact any agreement with the PBAC as sponsors would be cognisant of further price reductions in the post recommendation process." (Novartis Australia)

"AbbVie does not support this Option as a tool for enabling and expediting the previously elaborated on Options 4.1 "Discounted cost-minimisation" and 2.2 Streamlined pathways for cost-minimisation submissions. Any pricing offer and negotiation guidance framework should be with the procedural purpose of providing clear instruction and guidance with respect to pricing methods and improving transparency and certainty for Sponsors by outlining grey areas and

policy nuance that Sponsors may be unaware of. AbbVie's Consultation 1 response made the pragmatic recommendation to reintroduce a pricing methods manual as previously used prior to 2014 to ensure transparency and predictability of negotiated PBS prices. Furthermore, where there are different interpretations between Sponsors and the Department of Health and Aged Care (DoHAC) around the PBAC's pricing recommendations, there should be a pathway to clarify these matters with PBAC in an expedited manner." (AbbVie)

"Roche does not support the introduction of a pricing offer and negotiation guidance framework. Introducing another pricing and cost control mechanism will only result in unsupported lower prices being requested, such as the proposed funding eligibility for a streamlined cost-minimisation therapy, which will delay access to patients. Post-PBAC pricing negotiations are already conducted under a robust guidance framework informed by the PBAC, its sub-committee deliberations, and final PBAC recommendations. Additional frameworks will add unnecessary complexity and rigidity and further slow down the process." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

While there was support for this reform among this stakeholder group, it was noted that such guidance needed to have an appropriate level of flexibility and not be too prescriptive or narrow.

"It is impossible to judge the impact of this from the information provided. While guidance can be welcome, some overseas systems put a 'straitjacket' onto the negotiation process which may not reflect all the variables. Impact depends on the details." (Medical Technology Association of Australia)

Table 59. Post-listing re-assessment of health technologies – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	13%	38%	0%	50%	8
Pharmaceutical / Medical technology company	52%	33%	0%	0%	0%	14%	21
University or research sector	0%	0%	33%	33%	33%	0%	3
Industry association / Peak body	33%	17%	0%	50%	0%	0%	6
Clinician (or representative organisation)	0%	0%	100%	0%	0%	0%	1
Consulting	33%	33%	0%	0%	0%	33%	3
State / Territory government	0%	0%	0%	100%	0%	0%	2
Other	0%	0%	33%	67%	0%	0%	3

Patients, Consumers and Representative Groups

Those patient and consumer groups providing additional comments on this specific reform were supportive, albeit with a strong need to involve consumer and patient groups in any reassessment process.

"This option is promising as it potentially allows earlier access to health technologies. Post-listing re-assessment should also include measures for community health outcomes in this framework, developed with input from consumer expertise." (Mito Foundation)

Pharmaceutical / Medical Technology Companies

Most Pharmaceutical / Medical Technology Companies questioned the value of this reform option, suggesting the mechanisms for government to disinvest were already well provided for.

"The 'explicit disinvestment framework' should be re-framed as an explicit re-assessment framework with clear decision criteria against which technologies will be considered for continued funding or disinvestment. MSD welcomes a 'systematic and enhanced, rapid' program to provide advice on funding and disinvestment of technologies. However, the focus on 'an explicit disinvestment framework' should be broadened to encompass funding of cost-effective technologies where there is unmet clinical need. The Commonwealth already has measures in place to 'disinvest' from technologies, such as statutory price cuts and a rapid post-market review framework updated in February 2024." (MSD Australia)

"Disinvestment considerations are reflected in current post-market review arrangements, as well as statutory price reductions and reference pricing. Any developments in a program that provides disinvestment advice to Government should incorporate greater rigour, or at least match - the efforts, stakeholder engagement, evidence requirements and HTA methods employed to support recommendations of PBS listing. This includes following high level evidence

principles outlined in the clinical evaluation section of the Options paper, such as favouring RCTs. Criteria for disinvestment decisions need to be explicit, stakeholders must be consulted with enough time to provide relevant information or data analyses during disinvestment considerations and the reasons for disinvestment decisions must be clearly communicated to all relevant stakeholders. The framework needs to differentiate between treatments that are listed conditionally and those that follow standard entry pathways." (AstraZeneca)

"A disinvestment framework is unnecessary. Delisting from PBS should continue to a disallowable instrument so that parliamentary scrutiny and decision making remains possible." (Alexion)

"There is currently no need for the post-listing reassessment of health technologies. The regular application of DUSC reviews in the post-listing environment is sufficient to determine the use of the drugs as per the agreed usage. To add in reassessment criteria would be a disincentive for investment." (Novartis Australia)

"This option is not required. There is already a post-market review framework which was recently updated in consultation with industry and other stakeholders." (Eli Lilly Australia)

"Bayer is not supportive of this option, as it is not clear why it is required given the existence and recently updated (February 2024) rapid post-market review framework." (Bayer Pharmaceuticals ANZ)

"Roche does not support the proposed option of rapid post-listing reassessments of health technologies. The well-established and recently updated post-market review process already provides a systematic approach to monitoring medicines following PBS listing to inform decision making relating to ongoing access and subsidy." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholders were commonly supportive of a disinvestment mechanism in principle as a means of optimising limited public resources.

"This is particularly important for those technologies on "bridging" funding but could also be extended to fast-moving areas of medicine development i.e. where there are multiple treatment options for one indication. The time period for review might differ depending on the disease area and rate of technological development." (Adelaide Health Technology Assessment)

"By periodically reviewing health technologies after their initial evaluation, this program enables timely updates to funding or purchasing decisions based on evolving evidence and changing clinical needs. Additionally, the incorporation of an explicit disinvestment framework ensures that resources are allocated efficiently, redirecting funding from technologies that no longer provide significant value to those that offer greater benefit." (Society of Hospital Pharmacists of Australia)

Others had concerns around the resource impost of a fixed cycle of post-funding reviews, noting that reviews should be undertaken for a specific reason as opposed to any fixed cycle or timeframe.

"Routine reassessment regardless of identified need uses up a lot of resources potentially unnecessarily. Reviews should be based on identified rationale. It is not clear how this would improve patient access. However, if this is done it should be based on clear principles including transparency and consultation." (Medical Technology Association of Australia)

Table 60. Approaches for managing uncertainty - bridging funding coverage for earlier access to therapies of likely HATV and HUCN – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	22%	56%	22%	9
Pharmaceutical / Medical technology company	5%	5%	20%	40%	5%	25%	20
University or research sector	0%	0%	33%	33%	33%	0%	3
Industry association / Peak body	0%	0%	0%	86%	14%	0%	7
Clinician (or representative organisation)	0%	0%	0%	0%	100%	0%	1
Consulting	0%	0%	0%	67%	0%	33%	3
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	0%	50%	50%	0%	0%	4

Patients, Consumers and Representative Groups

Consumer and patient groups were positive in their support for this proposed reform, welcoming any mechanism that can help bring medicines to market in areas of high unmet clinical need.

"APAA support bridging funding coverage for earlier access to specific therapies such as those that seek to address High Unmet Clinical Need, in particular for paediatric access to medicines." (Australian Patient Advocacy Alliance)

"CHF supports legislation that enables conditional listings on the PBS for therapies of High Added Therapeutic Value and High Unmet Clinical Need. This will ensure that price negotiations between sponsors and the government do not cause unnecessary delays to consumers in accessing life-saving medication." (Consumers Health Forum of Australia)

"We strongly support this option that will enable provisional access to new therapies." (Rare Voices Australia)

Some stakeholders noted the importance of transparent processes, especially around pathways to the removal of bridging funding.

"This can be the difference between life and death for some patients and could work well in combination with triaged assessment approaches. However, transparency is needed on what

health technologies and diseases will qualify for this. Additionally, the bridging funding process needs to be carefully managed so that pathways to withdrawal of funding are clear. There is a risk that a subsequent recommendation to not fund a product, or to fund for a different population, could result in political pressure being applied to HTA decision makers. This could undermine the strength of Australia's HTA process." (Mito Foundation)

"Bridging funding should be established through a capped special funding program (separate and distinct from the PBS special appropriations) or legislate to enable conditional listings on the PBS." (Childhood Dementia Initiative)

The Collaborative Consumer Group Response submission also believed that measures for addressing uncertainty was critical, but that they would like to have seen *"options that more clearly address potential gaps created by joint Commonwealth and State funding of technologies and the reliance on the NHRA to address these gaps in a timely and equitable manner for patients. In practice this means people in some states have access to advanced therapies while others either have no access or are required to travel interstate to get access – this must be addressed as a matter of urgency."* (Collaborative Consumer Group Response group submission).

Pharmaceutical / Medical Technology Companies

There was also support for this reform among Pharmaceutical / Medical Technology Companies, albeit with some concerns identified in relation to the time-based restrictions on eligibility and the challenges of data collection pertaining to small patient cohorts (e.g. those suffering rare diseases).

"Bridging funding has been successfully introduced in other markets and is a welcomed option. It is important that this option is implemented as part of a comprehensive set of changes that accelerate access to all innovative medicines." (Eli Lilly Australia)

"Alexion in principle supports the options for bridging funding, however considers:

- *A six-month requirement from first international registration is unworkable and would severely restrict the effectiveness of the scheme and should be removed.*
- *Sponsors should be able to apply outside the constraints of a priority list where horizon scanning has not identified a particular critical and innovative therapy that would benefit Australian patients.*
- *Any bridging scheme needs to recognise the intrinsic difficulties of data collection for rare diseases with small patient cohorts and long periods of treatment. This is quite different to the capacity for data collection in other health fields like oncology.*
- *Greater clarity needs to be provided in relation to risk-sharing to ensure that the bridging fund avoids the disincentives with existing managed access programs (both in Australia and other jurisdictions)."* (Alexion)

Others wanted more detail on the scope of this measure before being able to support it.

"We welcome bridging funding to ensure HATV and HUCN achieve the earliest access for patients. In principle the high-level steps outlined are reasonable, however there is no detail around the criteria of what would qualify to be accepted through this pathway. Also, sometimes a novel medicines mechanism of action may not be included in the horizon scanning when it should be included - so is there an opportunity for sponsor companies to put forward technologies to be added to the HUCN priority list? The opportunity for managing uncertainty should include collaboration and discussion between the PBAC committees as well as the sponsor company to ensure equitable and faster access to technologies, and in keeping with recognising the value of medicines." (Antengene Australia)

"The proposed program would benefit from additional consideration of the following: 1) Applicants should be allowed an opportunity to justify that their technology addresses a HUCN. A central body identifying areas of HUCN may not capture all opportunities in a priority list or via horizon scanning. Applicants, who include experts intimately involved in their area of clinical need, may identify opportunities that a central body would not. 2) It is my understanding that under HATV, therapies or therapeutic value would also include diagnostic devices. Clarification on this point would be appreciated. 3) The great majority of medical device manufacturers in Australia seek first regulatory clearance from a comparable overseas regulator, which expedites TGA review. Therefore, parallel application to MSAC and TGA is of little benefit since the MSAC review would continue long after TGA review. It is recommended that the MSAC application is allowed at the time of application to a comparable overseas regulator, while maintaining that any MBS listing would require ARTG listing first. 4) I am unclear how capped reimbursement would apply to medical devices. The cost-benefit decision to purchase capital medical equipment is directly impacted by the availability of reimbursement. If a hospital purchases equipment dependent on receipt of the reimbursement, what would happen when the cap is met? How would they know?" (Anonymous submission)

"This proposal doesn't refer to the use of bridging funding for provisional listings which appears to be a key gap, although this may be inferred. Determination of what is meant by parallel processing is required. In reality PBAC submissions require cost effectiveness analyses that rely on the readout of the pivotal trial. This proposal requires further work to clarify key questions and gaps." (Pfizer)

"Roche supports either establishing bridging funding coverage or enabling conditional listings on the PBS for earlier access to therapies of HATV and HUCN. Roche would seek further consultation on this option with further detail shared on the key stages of the program being considered particularly the early identification/priority list designation, eligibility requirements, HTA application approaches, data collection, and final assessment stages. It is also important to ensure that any new funding mechanisms do not result in further delays, or create inequities to patient access." (Roche Products)

"AZ believe that revised MAP guidance and policy arrangements need to be developed that encourage their utilization and that any re-design should not introduce further complexity or create perverse incentives. The UK Cancer Drugs Fund could be used as a model, however, claw-back, patient continued access issues and the assessment of whether a treatment is effective need consideration. The eligibility requirement for the MAP pathway proposed in the Options paper that treatments be lodged with the TGA and PBAC submissions simultaneously and within 6 months of receiving first international regulatory approval will limit uptake. AZ

support development of a new managed access model in Australia through formation of a working group." (AstraZeneca)

"While (AbbVie is, in-principle, supportive of the establishment of a bridging funding program, (AbbVie strongly opposes any legislative change that would permit the PBAC to make conditional recommendations. (AbbVie proposes that the co-development of a workable framework for Managed Access Programs (MAPs) to ensure they are more feasible and implementable would be an appropriate alternative with legislative change not required, and instead could be managed within a Deed. (AbbVie recognises that the establishment of a bridging funding program could present a potential opportunity to improve time to access for specific health technologies by closing the funding gap between provisional TGA approval and PBS listing by allowing PBAC submissions to be made earlier based on Phase I/II data. Industry/Sponsors must be involved as a key partner in the co-design of any bridging funding program to ensure that the process of applying for, granting of and transition to and from bridging funding for specific products takes into account supply chain timing and the transition of trial patients and does not have any negative or unintended consequences for stakeholders." (AbbVie)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Many of these stakeholders were supportive of the concept but wanted more information on how the program would work in practice.

"Of particular interest to oncologists is the approach outlined for managing uncertainty, i.e. bridging funding coverage to enable earlier access to therapies of likely HATV and HUCN. The provision of interim bridging funding and a mechanism to allow assessment of value post-listing would be one important mechanism to gain earlier access for patients however the proposed mechanism appears restricted to a small number of applications, the detail around how these technologies would be selected for this funding is not clear and this discussion appears to be part of an aspirational goal rather than one which is ready for immediate discussion and implementation. This is unfortunate. The document appears to suggest that ongoing advocacy for further reforms is needed to enable more meaningful change." (Clinician)

"By creating a separate funding program or enabling conditional listings on the PBS, this option allows for expedited access to therapies that demonstrate significant potential but require further evaluation." (Clinician)

Table 61. Approaches for managing uncertainty - revised guidance on the uses of different managed entry tools – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	14%	14%	43%	29%	7
Pharmaceutical / Medical technology company	5%	10%	20%	35%	5%	25%	20
University or research sector	0%	0%	33%	67%	0%	0%	3

Industry association / Peak body	0%	0%	0%	100%	0%	0%	6
Clinician (or representative organisation)	0%	0%	0%	0%	100%	0%	1
Consulting	0%	0%	0%	100%	0%	0%	3
State / Territory government	0%	0%	0%	0%	0%	100%	1
Other	0%	0%	25%	75%	0%	0%	4

Patients, Consumers and Representative Groups

Consumer and patient groups were positive towards this reform, especially those representing patients in often dire need of access to higher cost therapies.

"We strongly support this option. Managed entry programs should be strongly linked with systematic data collection of RWE/observational evidence to address gaps in evidence for indications for small patient populations such as in rare disease. It would be strengthened by measures to ensure that information about managed entry programs of all types is transparent and accessible. Patients currently experience significant equity in special and managed access schemes and this could be addressed by ensuring increased visibility and transparency of these schemes." (Rare Voices Australia)

The Collaborative Consumer Group Response submission also supported this option and they believed *that "approaches to managing uncertainty that enable earliest possible access to HAVT/HUCN to technologies"* was one of the strengths of this section of the reform options paper. (Collaborative Consumer Group Response)

Pharmaceutical / Medical Technology Companies

Many Pharmaceutical / Medical Technology Companies indicated in-principal support for this reform, albeit with a need for additional consultation and co-design of any new guidelines on managing uncertainty across the HTA process. The equitable sharing of risk was a common requirement identified across multiple submissions.

"Changes to increase uptake of managed entry are welcomed. Management of uncertainty is an issue that extends across the clinical, economic and budget impact aspects of a HTA submission. A framework for managing uncertainty and ensuring the risk is genuinely shared rather than passed in entirety to the Sponsor is critical." (Eli Lilly Australia)

"The development of any guidelines should must be undertaken in collaboration with stakeholders." (Pfizer)

"Roche supports revised guidance and policy arrangements to encourage the creative proposition and use of managed entry tools and instruments. Roche would seek further consultation on this option with further detail shared on what creative parameters would be tolerated, and how implementation may be achieved. Roche encourages the Review Committee to also consider revising policy arrangements to support more creative use of existing risk- sharing arrangements and SPAs, and how this also may seek to benefit patient access." (Roche Products)

"Revised tools for managing uncertainty sounds encouraging, but we would need to understand how this was to be implemented." (UCB Australia)

"A clear and definitive plan for any bridging funding program must be articulated which also includes information about how therapies are identified and importantly what happens once the capped bridging funding ends is required." (Novartis Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholders were generally either supportive or requiring more detail on the proposed reform.

"By encouraging creative propositions and utilisation of managed entry arrangements, this option promotes collaborative engagement between sponsors and decision-makers to address uncertainty constructively." (Society of Hospital Pharmacists of Australia)

"Clinicians will support novel mechanisms to bring new treatments to our patients earlier. This would appear to be a promising mechanism and improvements can be made on existing programmes." (Clinician)

"This recommendation is a little vague. I wasn't quite clear on what was being suggested." (Adelaide Health Technology Assessment)

Comparing the 4.1 Alternative options

Table 64. Reform option you think offers greatest scope to address the issues identified in consultation to date by stakeholder type

	Alternative option 1: In conjunction with options for proportionate assessment of cost-minimisation submissions, require offers of a lower price for health technologies that provide no added benefit	Alternative option 2: In conjunction with options for proportionate assessment of cost-minimisation submissions, incentivise offers of a lower price for health technologies that provide no added benefit.	Neither of these	Sample size
Patient or consumer (or representative organisation)	0%	60%	40%	5
Pharmaceutical / Medical technology company	0%	0%	100%	22
University or research sector	33%	33%	33%	3
Industry association / Peak body	0%	20%	80%	5
Clinician (or representative organisation)	-	-	-	0
Consulting	0%	33%	67%	3

State / Territory government	-	-	-	0
Other	0%	33%	67%	3

In line with feedback above, there was universal rejection of both options among pharmaceutical stakeholders. There was far less engagement on this question across other stakeholder groups, with many of those providing a response choosing the 'neither of these' option.

4.2. Approaches to incentivise development of products that address antimicrobial resistance (AMR)

Table 65. 4.2. Approaches to incentivise development of products that address antimicrobial resistance (AMR): How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	67%	0%	33%	3
Pharmaceutical / Medical technology company	0%	45%	36%	9%	9%	11
University or research sector	0%	0%	0%	100%	0%	1
Industry association / Peak body	0%	25%	75%	0%	0%	4
Clinician (or representative organisation)	0%	100%	0%	0%	0%	2
Consulting	-	-	-	-	-	0
State / Territory government	-	-	-	-	-	0
Other	0%	0%	33%	33%	33%	3

Patients, Consumers and Representative Groups

No patient or consumers groups provide notable comments on this specific reform option.

Pharmaceutical / Medical Technology Companies

Those companies providing comment on this reform option suggested this was a long-standing challenge and that solutions have already been identified through alternate means.

"Australia needs the rapid establishment of a reimbursement model that de-links revenue from volume. Australia's response should recognise the value of the antimicrobial to the health system. The information about reforms that address the long-standing problem is already available and implementation of a new approach must be undertaken with urgency." (Pfizer)

"Discussions regarding the reimbursement models and options to incentivise and ensure fast access to AMR have been conducted over the past years, with consensus reached for several

*options. We suggest that rather than run a different workshop, the explored solutions should be implemented in the short term." (*UCB Australia)

Table 66. HTA Fee exemptions for products that address AMR – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	50%	0%	50%	2
Pharmaceutical / Medical technology company	0%	0%	9%	55%	18%	18%	11
University or research sector	0%	0%	100%	0%	0%	0%	1
Industry association / Peak body	0%	0%	25%	75%	0%	0%	4
Clinician (or representative organisation)	0%	0%	0%	50%	50%	0%	2
Consulting	-	-	-	-	-	-	0
State / Territory government	-	-	-	-	-	-	0
Other	0%	0%	100%	0%	0%	0%	2

Patients, Consumers and Representative Groups

No patient or consumers groups provide notable comments on this specific reform option.

Pharmaceutical / Medical Technology Companies

Many Pharmaceutical / Medical Technology Companies were supportive of this specific reform.

"As part of a package of initiatives, this will help in improving access to anti-microbials in Australia. Importantly, access to HTA fee exemptions should not be restricted to the WHO priority list as this could exclude pathogens particular to Australia (such as Buruli ulcer) that are not of global concern but are of particular concern locally. The fee exemption should apply across all approval decisions through the TGA and PBAC to ensure any impediments to access are avoided. While the current orphan drug designation solves for some of these issues the proposal appears to go further which is a positive step. Implementation must occur without further delay." (Pfizer)

"BiomeBank welcomes the Reference Committees acknowledgement of high-cost burden and we believe the fee exemption should cover the entire lifecycle of the therapy. BiomeBank has experience as a small biotech company bringing a product to a relatively small but important market (antibiotic refractory C. difficile infection). The Marketing Authorisation and Reimbursement submission costs are a barrier to small innovative companies such as ours and the ongoing regulatory costs place a significant burden on our ability to produce and maintain economic viability." (BiomeBank)

"Roche supports mechanisms that incentivise further research and development through to patient access of new antimicrobial therapies. Whilst fee exemptions are one option, further incentives should be considered, and Roche would welcome further consultation and detail on what these mechanisms could look like." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholders were equally supportive of this proposed reform.

"This approach recognises the critical importance of addressing AMR as a global public health priority and acknowledges the unique challenges associated with developing antimicrobial therapies. By exempting HTA fees, this option encourages investment in research and development of new antimicrobial products, fostering innovation in this crucial area of healthcare." (Society of Hospital Pharmacists of Australia)

Table 67. HTA Policy and Guidance changes for products that address AMR – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	0%	0%	100%	2
Pharmaceutical / Medical technology company	0%	0%	18%	55%	9%	18%	11
University or research sector	0%	0%	100%	0%	0%	0%	1
Industry association / Peak body	0%	0%	25%	75%	0%	0%	4
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	2
Consulting	-	-	-	-	-	-	0
State / Territory government	-	-	-	-	-	-	0
Other	0%	0%	100%	0%	0%	0%	2

Patients, Consumers and Representative Groups

No patient or consumers groups provide notable comments on this specific reform option.

Pharmaceutical / Medical Technology Companies

There was support for this reform, albeit with calls for further consultation and co-design of any new policy or process.

"BiomeBank welcomes review of HTA policy to enable flexibility and improve pathways to funding/reimbursement to enable newly developed technologies to reach consumers in a more efficient and rapid manner." (BiomeBank)

"Roche agrees with the Review Committee's recommendation to conduct workshops to provide further detail and clarity on possible options to incentivise patient access to new antimicrobials. Roche would also recommend that the Department of Health and Aged Care make the existing work program more transparent to stakeholders, with clear timeframes and models where stakeholders can be further involved in co-design and consultation." (Roche Products)

"These initiatives are important and should be pursued, however, we are concerned about a risk that this work becomes a roadblock that causes further delays in access to novel anti-infectives. This work should be done in parallel with creation of pull incentives via a novel funding arrangement that delinks value from volume as further consideration of HTA policy and guidance should not prevent an urgent response to the urgent problem of lack of access to anti-infectives. Consideration of HTA policy and guidance should be linked to firm timelines and implementation goals to ensure the intended outcome of improved access to anti-infectives is achieved as quickly as possible." (Pfizer)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

One stakeholder provided comment on this specific reform, noting the importance of aligning evaluation methods with the specific challenges of AMR.

"This option promotes a proactive and collaborative approach to addressing AMR through HTA policy and guidance changes. By aligning evaluation methods with the unique challenges posed by AMR, this approach incentivises the development and market availability of antimicrobial products, ultimately contributing to efforts to combat antimicrobial resistance and safeguard public health." (Society of Hospital Pharmacists of Australia)

Table 68. Funding and reimbursement-related changes to support availability of antimicrobials – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	50%	0%	50%	2
Pharmaceutical / Medical technology company	0%	0%	18%	55%	9%	18%	11
University or research sector	0%	0%	100%	0%	0%	0%	1
Industry association / Peak body	0%	0%	25%	75%	0%	0%	4
Clinician (or representative organisation)	0%	0%	0%	50%	50%	0%	2
Consulting	-	-	-	-	-	-	0
State / Territory government	-	-	-	-	-	-	0
Other	0%	0%	100%	0%	0%	0%	2

Feedback across all groups was consistent with that reported above.

4.3. Understanding the performance of health technologies in practice

Table 69. 4.3. Understanding the performance of health technologies in practice: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	62%	31%	0%	8%	13
Pharmaceutical / Medical technology company	0%	53%	47%	0%	0%	15
University or research sector	0%	33%	33%	33%	0%	3
Industry association / Peak body	0%	86%	14%	0%	0%	7
Clinician (or representative organisation)	0%	100%	0%	0%	0%	1
Consulting	0%	67%	33%	0%	0%	3
State / Territory government	0%	0%	100%	0%	0%	2
Other	0%	50%	25%	25%	0%	4

Patients, Consumers and Representative Groups

Patient and consumer groups were supportive of these reform options, noting that there is a desire to move quickly in establishing protocols for the appropriate capture and reporting of RWD and RWE in appropriate format and contexts.

"I have stated mostly because although the options are good ones, they are not new and have been discussed before so of themselves they are clearly not totally the answer. There must be an appetite to implement and support all stakeholders. The exploration of why these important pieces have not already been in place must underpin the way forward." (Genetic Support Network of Victoria)

"The mechanisms listed in this section are important enablers to improving outcomes for people living with rare diseases especially. All efforts to establish these mechanisms should consider existing government initiatives and possibly patient organisations' work to ensure efficiency and standardising approaches across all stakeholders." (Mito Foundation)

"RVA supports all options listed in this section as mechanisms to provide earliest possible access to health technologies for HUCN/HAVT. Such measures will assist in resolving uncertainty and informing ongoing access decisions. Any investment in registries must consider broader uses in the health system and be aligned with existing governing initiatives, including clinical quality registries funding and the Action Plan. The Action Plan identifies systematic rare disease data collection as a key enabler for improved health outcomes for people living with a rare disease. A broader approach would leverage funding allocations and reduce duplication of effort." (Rare Voices Australia)

"CHF supports the optimisation of access and use of Real World Evidence (RWD), and in particular an approach that centres consumers, community engagement and co-design. CHF supports the creation of a whole-of-government data infrastructure that is transparent and streamlined, and that is harmonised using international standards. On the other hand, as mentioned earlier in this submission, this must happen in a way that safeguards the privacy and safety of consumer data. Measures should also be put in place to prevent consumer-generated data to be used for financial gain. Consumers are adamant that they are happy to release data for altruistic purposes. The use of such data for financial profit however is completely unacceptable for them. Legislators must not shy away from this requirement, and ensure that there are clauses in place preventing this from happening." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

Many pharmaceutical and medical technology company stakeholders were supportive of this proposed reform, albeit with a need for further discussion of data collection infrastructure and who bears the costs of the collation and reporting of such data.

"The improvements to RWD and RWE if implemented would be welcomed. It will be critical that medicines manufacturers have a mechanism for accessing the data." (Eli Lilly Australia

"Roche supports an open and trusted health-data ecosystem, as well as, the secondary use of health data to increase the value of currently collected data, and in appropriate circumstances within the HTA lifecycle. In turn, Roche supports optimising access to and use of RWD in HTA, and increasing confidence, awareness, and acceptance of cross-jurisdictional and cross- sectoral RWD access and use in HTA. This should also be coupled with a data infrastructure strategy and implementation plan, and methods and guidance frameworks. Roche notes that the use of RWD to understand the performance of health technologies in the Australian setting through the collection of utilisation and outcome data is best placed for provisionally listed health technologies or areas of significant uncertainty. Roche recommends that further work on RWD is aligned to existing work currently being undertaken by the TGA." (Roche Products)

"The formation of a multi-stakeholder working group to design guidelines and develop systems to increase use of RWE in HTA would largely have a positive impact. It is not clear how registries should be used to facilitate the collection of outcome data for provisionally listed technologies. Stakeholder engagement in RWE guideline development will help overcome methodological issues" (AstraZeneca)

"Building a clear picture of the RWE gaps in Australia and the requirements for developing RWE enabling systems, pathways and evaluation in Australia through a multi-stakeholder advisory group will be useful. Proposals to develop data infrastructure and methods, as well as the development of a guidance framework are also welcomed with recognition that these objectives will require a coordinated multi-stakeholder approach. Collection of utilization and outcome data for provisionally listed health technologies is welcomed. While generation of data from existing registries is preferable, it is noted that such registries will not be available for many health technologies. Even where clinical registries do exist, they may not be fit-for-purpose for capturing the data required for HTA purposes. Alternative options for collection of utilization and outcome data for CED arrangement will also be required. Critically, all of these positive options for reforming RWE infrastructure, methods and access in Australia must also be coupled with

greater acceptance of RWE to support decision-making, to deliver on the shared goals of the HTA Review." (Pfizer)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

A number of these stakeholders were positive in their reaction to this specific reform.

"A research agenda is needed to evaluate how these reforms can be implemented, and once implemented evaluate whether they have changed practice. (positive and negative)." (Health Services Research Association of Australia and New Zealand)

"The measures proposed are overwhelmingly welcome subject to the detail of implementation. However, MedTech and digital health needs to be specifically considered in this context as well. We note that digital health and MedTech with digital health connectivity is unique in that it can collect some of its own data relating to performance following uptake." (Medical Technology Association of Australia)

Others highlighted concerns regarding RWE – if not managed within an agreed set of protocols – potentially lowering the standard of evidence needed to prove efficacy in practice.

Table 70. Oversight – reforms to optimise access to and use of RWD in HTA – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	31%	54%	15%	13
Pharmaceutical / Medical technology company	0%	6%	19%	44%	13%	19%	16
University or research sector	0%	0%	33%	33%	0%	33%	3
Industry association / Peak body	0%	0%	0%	63%	25%	13%	8
Clinician (or representative organisation)	0%	0%	0%	50%	0%	50%	2
Consulting	0%	0%	33%	33%	33%	0%	3
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	0%	33%	33%	33%	0%	3

Patients, Consumers and Representative Groups

The consumer and patient representative groups reacted favourably towards this reform.

"Regarding understanding the performance of health technologies in practice (4.3)— Painaustralia supports reforms to optimise access to and use of real-world data (RWD) in HTA. The proposed establishment of a multi-stakeholder advisory group, reporting to government, to co-design and oversee the development and implementation of enabling systems, pathways,

evaluation, and research to optimise access to and use of RWD in HTA would be a constructive measure." (Painaustralia)

"This is particularly important in managed entry and provisional approval options, as this data will be crucial to understanding clinical effectiveness of treatments for small patient populations where there are gaps in existing data." (Rare Voices Australia)

"This approach should centre consumer and community engagement and co-design, leverage and integrate existing international activities and guidelines, incorporate Australian context and evidence, and fine tune responses and messages specific to HTA." (Childhood Dementia Initiative)

"Such an approach should centre consumer and community engagement and co-design, integrate existing international activities and guidelines, and incorporate local context and evidence." (NeuroEndocrine Cancer Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

While these stakeholder groups viewed this reform option positively, several stakeholders noted the importance of capacity building across all stakeholders would be essential in optimising collection and use of RWE in HTA.

"Establishing a multi-stakeholder advisory group to optimise access and use of RWD in HTA represents a strategic initiative to strengthen evidence-based decision-making and support continuous improvement in healthcare delivery. By bringing together diverse stakeholders, including healthcare professionals, researchers, policymakers, and patient representatives, this approach ensures that RWD is leveraged effectively to inform HTA evaluations." (Society of Hospital Pharmacists of Australia)

"Sounds like a very sensible idea. HTA practitioners need to get better at this. In addition to multi-stakeholder, I would be inclined to include multi-disciplinary. Economists in other disciplines use real world data all the time to make funding decisions - we can learn from that." (THEMA Consulting)

"IQVIA agrees with the importance of establishing a multi-stakeholder advisory group to government to guide next steps for optimising access and use of RWD in HTA. It will be critical for this advisory group to contain a well-rounded mix of stakeholder perspectives, with adequate representation from the commercial sector, in order to produce realistic and implementable recommendations that appropriately account for known operational challenges and limitations (including budget and timeline constraints)." (IQVIA)

Table 71. Develop a strategic approach to increase confidence, awareness, and acceptance of cross-jurisdictional and cross-sectoral RWD access and use in HTA – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	8%	31%	54%	8%	13
Pharmaceutical / Medical technology company	0%	6%	13%	44%	19%	19%	16
University or research sector	0%	0%	33%	67%	0%	0%	3
Industry association / Peak body	0%	0%	0%	75%	25%	0%	8
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	2
Consulting	0%	0%	0%	67%	33%	0%	3
State / Territory government	0%	0%	0%	100%	0%	0%	2
Other	0%	0%	33%	33%	33%	0%	3

Patients, Consumers and Representative Groups

This reform options were broadly supported across consumer and patient representative groups, albeit with some noting the importance of both privacy and data security – especially when dealing with small/very small patient cohorts.

"This is particularly important in managed entry and provisional approval options, as this data will be crucial to understanding clinical effectiveness of treatments for small patient populations where there are gaps in existing data." (Rare Voices Australia)

"Essential further strategies are applied so Australia may continue to develop and enhance systems that ensure privacy protections and data security." (NeuroEndocrine Cancer Australia)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies were broadly supportive, with many calling for a co-design phase to ensure the approach was appropriately informed and fit for purpose.

"Alexion is supportive of this option and recommends its development by the committee overseeing the development and implementation of systems to optimise access to RWD/RWE." (Alexion)

"A co-design approach with consumer and community is welcomed, however we need to understand first what data we are collecting and how it will be used. Integrate existing international activities and guidelines - there is no detail what this means in terms of context and implementation, so we are unable to support this at this time." (Antengene Australia)

"Roche supports further work on the strategic approach to increasing RWD acceptability and use in Australian evaluations and decision-making frameworks. Further consultation and clarity is needed on the mechanisms, policy, processes and frameworks that would enable this, with the need for industry collaboration and input into the co-design." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

While many of these stakeholders were supportive, some identified key logistical challenges and risks that will need to be addressed.

"Developing a strategic approach to increase confidence, awareness, and acceptance of cross-jurisdictional and cross-sectoral RWD access and use in HTA supports evidence-based decision-making and strengthens the integrity of HTA evaluations. By engaging stakeholders, leveraging international expertise, and prioritising data privacy and security, Australia can optimise the use of RWD to enhance understanding of health technology performance and improve healthcare outcomes." (Society of Hospital Pharmacists of Australia)

"Suggest this is done with careful consideration to the quality standards of data." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

Table 72. Data infrastructure – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	46%	46%	8%	13
Pharmaceutical / Medical technology company	0%	6%	6%	50%	19%	19%	16
University or research sector	0%	0%	33%	33%	33%	0%	3
Industry association / Peak body	0%	0%	0%	88%	13%	0%	8
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	2
Consulting	0%	0%	0%	67%	33%	0%	3
State / Territory government	0%	0%	0%	100%	0%	0%	2
Other	0%	0%	33%	33%	33%	0%	3

Patients, Consumers and Representative Groups

Patient and consumer organisations were broadly supportive, albeit with a need to ensure current data holdings and networks are fully scoped/mapped first to avoid duplication of effort.

"Needs to include Indigenous data governance." (NACCHO)

"Data infrastructure options should align with broader government investments in clinical registries (i.e. clinical quality registry investment) to reduce duplication and leverage existing investment." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

While Pharmaceutical / Medical Technology Companies were broadly supportive, some noted a need for legislative change and greater alignment of data sharing practices across jurisdictions. Some also queried how any new data infrastructure would be funded.

"Strongly support - encourage legislative changes that would permit secondary use of dataset by providers/industry. This could include providing a definition of 'for public benefit', allowing better cooperation from data custodians and speed up required ethics approvals." (Biogen)

"The ambition is welcomed; however, this is likely to take many years, resources and funding to implement. We need to consider having short-, medium- and long-term milestones and be clear about who and how this is implemented. It needs to be a flexible system to be able to change over time. Ultimately what data is collected, how it will be used and where it fits in the decision-making process needs much more detail in order to support." (Antengene Australia)

"Alexion is supportive of a national approach but for it to be successful, significant investment will be necessary to establish a national database where data can be collected and shared between federal and state health departments. For instance, currently Victoria is the only state that has data shared between the immunisation registry and GPs general health data sets." (Alexion)

"Roche supports the option to develop a whole of government data infrastructure and would welcome further consultation to ensure that industry's role in RWE is included as part of the co-design process." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholders were largely supportive, but commonly tended to appreciate the challenges of achieving this goal – especially in terms of cross jurisdictional agreement.

"Developing a robust data infrastructure for RWE in HTA improves the understanding of health technology performance and supports evidence-based decision-making." (Society of Hospital Pharmacists of Australia)

"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." (Omico)

"This is central to all of this topic in a country like Australia. And is a bigger topic than just HTA. Not only are we not necessarily very good at using and analysing RWD, we can't get it most of the time anyway! I learnt a lot about this issue at the ISPOR workshop in 2023

(https://isporac.org/event/ispor-australia-chapter-workshop-on-real-world-evidence/). It feels like we are in the pre-harmonised rail gauges era!" (THEMA Consulting)

"IQVIA agrees with the perspective that further investment is required to advance and future-fit Australia's infrastructure, processes and systems for enabling high quality RWE generation. Although a wide range of interesting Real World datasets existing in Australia today, they are largely fragmented in nature, and data access and linkage remain challenging. Importantly, the benefits of investing in data infrastructure are multi-factorial." (IQVIA)

Table 73. Methods development – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	8%	38%	46%	8%	13
Pharmaceutical / Medical technology company	0%	7%	20%	60%	0%	13%	15
University or research sector	0%	0%	33%	67%	0%	0%	3
Industry association / Peak body	0%	0%	0%	88%	13%	0%	8
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	2
Consulting	0%	0%	0%	67%	33%	0%	3
State / Territory government	0%	0%	0%	100%	0%	0%	2
Other	0%	0%	33%	33%	33%	0%	3

Patients, Consumers and Representative Groups

Patient and consumer groups were broadly supportive of the option put forward for methods development, but commonly noted the importance of working with these groups to co-design these methods to ensure they are fit for purpose for each specific disease or condition.

*"It is critical that tools to measure the performance of health technologies in practice are fit for purpose…It is critical that the right methods and tools are used to understand the performance of healthcare technologies in practice." (*The Australian Diabetes Alliance).

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies were supportive in general, but wanted further detail on how such methods would be established in practice. Some also expressed concern regarding how resource intensive this might be, both to establish and to maintain over the longer term.

"Roche recognises that the structures may take a number of years to effectively establish, given the scale and scope of potential change and the potential consequences, intended and

unintended, with respect to understanding the performance of health technologies in practice." (Roche Products)

"As there is no detail, we are unable to support this at this time. What constitutes best-practice methods? What data is standardised? Who is responsible and how often is data analytics used? Unfortunately, there are more questions than answers. The other key question is who makes up the multi-stakeholder committee? We would advocate that industry is part of this committee." (Antengene Australia)

"Alexion recommends the development of databases which collect data from clinicians in a similar manner to the existing approach where Services Australia records the approval for patients meeting the continuation criteria for existing therapies. A pilot program should be trialled with stakeholder agreement on what data should be collected and how this data should be interpreted, this should include the involvement of the PBAC, clinicians and pharmaceutical sponsors at the time of establishment and review." (Alexion)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholders were also commonly supportive, with similar calls to ensure existing knowledge and expertise is leveraged in subsequent development.

"Developing a coordinated approach to evidence development using best-practice methods for HTA improves the quality and relevance of evidence used in decision-making processes." – (Society of Hospital Pharmacists of Australia)

"Supported. Sponsors who may support the establishment of the RWD must declare any conflicts in the reach or influence of the data (collected, analysed, reported)." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

"As above per my response to the oversight advisory group. I think it would be important to include a multi-disciplinary approach to this." (THEMA Consulting)

Table 74. Develop Guidance framework – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	8%	38%	46%	8%	13
Pharmaceutical / Medical technology company	0%	6%	19%	63%	0%	13%	16
University or research sector	0%	0%	33%	67%	0%	0%	3
Industry association / Peak body	0%	0%	0%	88%	13%	0%	8
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	2
Consulting	0%	0%	33%	33%	33%	0%	3
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	0%	33%	33%	33%	0%	3

Patients, Consumers and Representative Groups

Consumer and patient organisations commonly indicated development of these guidelines would positively impact their organisations. One stakeholder noted the importance of leveraging existing RWD/RWE frameworks rather than developing entirely new guidelines or protocols.

"Essential that guidance on the use of RWD and RWE would occur with the oversight of the aforementioned advisory group, following the development of methods. Delays to approvals should not be due to delayed development of frameworks. As an interim, the FDA data standardisation framework adopted by the TGA may also be adopted to guide the use of RWD in HTA for subsidy decisions." (NeuroEndocrine Cancer Australia)

Pharmaceutical / Medical Technology Companies

These stakeholders were also supportive but re-iterated the need for industry engagement and co-design in moving this concept forward.

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholders also broadly supported this reform, albeit with a number of key caveats as identified below.

"Supported, with the caveat that registries do not prescribe clinical decisions and are not established to monitor clinical data. Clinical trials are the appropriately regulated manner in which to ensure ethical consent of participants, treatment and clinical decisions and prospective data collection and monitoring." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

"As stated in Section 1.2, IQVIA agrees with the recommendation to provide more explicit guidance on the types of RWD/RWE that would be most informative to the HTA decision-making

process. Interestingly, we have previously heard anecdotal feedback from industry that lack of clear guidance around what RWE will be accepted – and associated uncertainty around ROI – disincentivises investment in high-quality RWE generation for HTA submission purposes. Availability of an aligned set of guiding principles could help to address this barrier, resulting in an increase in the overall quantity and quality of evidence being submitted." (IQVIA)

Table 75. Collection of utilisation and outcome data for provisionally listed health technologies – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	8%	38%	46%	8%	13
Pharmaceutical / Medical technology company	0%	6%	25%	50%	0%	19%	16
University or research sector	0%	0%	33%	33%	33%	0%	3
Industry association / Peak body	0%	0%	0%	88%	13%	0%	8
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	2
Consulting	0%	0%	33%	67%	0%	0%	3
State / Territory government	0%	0%	100%	0%	0%	0%	1
Other	0%	0%	33%	33%	33%	0%	3

Patients, Consumers and Representative Groups

This reform option was strongly supported among patient and consumer groups, again with comments noting a desire to leverage existing national and international registries.

"This is particularly important in managed entry and provisional approval options, as this data will be crucial to understanding clinical effectiveness of treatments for small patient populations where there are gaps in existing data." (Rare Voices Australia)

"Essential that existing national and international registries should be used, where possible, to facilitate the collection of outcome data relating to provisionally listed technologies in a timely fashion. For ultra-rare diseases, international registries should be utilised. Prior to entry into any arrangements, the likelihood of obtaining new evidence to address areas of uncertainty should be considered." (NeuroEndocrine Cancer Australia)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies were also broadly supportive, and similarly called for alignment with existing registries. The significant issue of adequate resourcing was also raised across a number of stakeholder responses.

"We support the use of existing national or international registries to facilitate outcomes data collection relating to provisionally listed technologies in a timely manner. A challenge with most registry databases is the quality of the input (i.e. missing data points and how it is heavily resource intensive for data entry). In order to improve the data input, there can be additional costs involved for this resource. With any database, you want quality data in to get quality data out. We would advocate that there is government funding to support these registries to ensure they are adequately resourced so that they provide the outputs that are meaningful. Another important consideration is what data will be collected and at what point in the submission process will it be used, including the weighting of the data." (Antengene Australia)

"In principle, Alexion supports the creation of registries and databases to collect outcome data for provisionally listed therapies. However, the options paper suggests that sponsors would carry the cost of this activity. This would be an unreasonable cost burden for sponsors noting that such data collection could cost several million dollars to establish and maintain. Different registries collect different data and often it is difficult to have one registry encompassing all data sets (i.e. there is no one "best" registry). Therefore, an approach where a specific PBS registry is created may be the most efficient for this intended purpose." (Alexion)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholders were also supportive but raised a number of key issues warranting consideration if this concept is to be taken forward.

"The collection of utilisation and outcome data through existing registries supports evidence- based decision-making and strengthens the HTA process by providing timely and comprehensive insights into health technology performance in real-world settings." (Society of Hospital Pharmacists of Australia)

"Just also to note that not all relevant data will necessarily come from registries. Coverage with evidence development could also rely on trials that are underway and not yet mature, or on administrative data (such as usage, co-administration of medicines, etc). The type of data required to reduce the decision-making uncertainty would need to be specified at the inception of provisional listing and would need to involve the HTA evaluators and committee discussants with the closest understanding of the submission." (Adelaide Health Technology Assessment)

"Successful implementation of Coverage with Evidence Development (CED) agreements is contingent upon timely and accurate data collection and reporting. IQVIA agrees that efforts should be made to harmonise data collection efforts with relevant existing databases and minimise duplicate data entry to the degree possible, in order to maximise data completeness and quality. However, this does not necessarily mean that all CED data collection should de- facto occur through existing registries. Instead, we recommend considering the merits of all available data collection options and selecting the most fit-for-purpose approach on a case-by- case basis, depending on the individual therapy area and product-specific dynamics and data requirements." (IQVIA)

Section 5: Futureproofing our systems and processes

Stakeholders were invited to provide written comment on the reform options presented for futureproofing Australia's HTA systems and processes as per the table below (reproduced from the HTA Review's Options Paper).

Subject	Key option/s
5. Futureproofing Australia's systems and processes	
5.1. Proactively addressing areas of unmet clinical need and gaps in the PBS – a systematic approach encompassing five interdependent new mechanisms. This new activity would require methodological development, implementation planning, and adequate resourcing including joint investment across stakeholder groups (see Figure 1).	
Development of a priority list	1. A priority list of areas of HUCN to be developed and regularly reviewed and updated in partnership between clinicians, patients and patient organisations, and community. 2. In line with the priority reforms under the National Closing the Gap Agreement 2020 between all Governments and the Coalition of Peaks, a sub-set of the priority list will be developed in partnership with ACCHSs for the priority areas of HUCN for First Nations peoples. 3. The list should include consideration of surveillance of AMR to identify new microbes developing resistance to current available treatments, and surveillance of vaccine preventable diseases.
Identifying therapies to meet priority list (horizon scanning)	1. An active horizon scanning process to be developed to identify therapies with promising HATV for indications on the priority list (this could include new therapies or new patient indications for the 'repurposing' of existing therapies). 2. This list is to include a mechanism for partnership with ACCHSs to ensure First Nations people's health outcomes and health equity is appropriately reflected. 3. This list would include technologies that do not have market authorisation in Australia as well as technologies where there is evidence they could be repurposed for new indications. *Note: See separate section on options for horizon scanning for further information and additional preferred options considered by the Reference Committee relating to horizon scanning.*

Early assessment and prioritisation of potentially promising therapies	Implement a system to assess and prioritise the therapies identified through horizon scanning with the goal of understanding which therapies represent important advances (HATV) in areas of HUCN.
Proactive submission invitation and incentivisation	After a therapy identified through horizon scanning has been prioritised through the early assessment, the Government could proactively request a sponsor submission. Incentives for the sponsor to bring a submission forward could include: • fee waivers • case management • priority pathway • potential for access to provisional funding programs (subject to HTA committee recommendation) (see Approaches for managing uncertainty) The sponsor would have a defined period to notify the Government of their intention to accept the offer (4-6 weeks) and then will have to make a submission to the PBAC (and application to the TGA if applicable) within a pre-defined time period.
Early PICO scoping	For therapies where the sponsor has accepted proactive submission invitation, early PICO scoping including identification of implementation requirements and challenges to occur (this could happen contemporaneously to the sponsor developing their submission).

5.2. Establishment of horizon scanning programs to address specific informational needs within HTA and the health system

Horizon scanning for advanced therapies (including high cost, HSTs funded through the NHRA) and other potentially disruptive technologies	Structured horizon scanning process: 1. Consistent with the NHRA mid-term review recommendation 29: A structured horizon scanning process should be established for HST's, with involvement of all jurisdictions, and with input from relevant stakeholders, including but not limited to the National Blood Authority, Organ and Tissue Donation Authority, HTA Advisory Committees (currently PBAC and the Medical Services Advisory Committee (MSAC)) to support forward planning and priority setting. 2. This should be done in partnership including Commonwealth, State and territory governments, and industry and on a cost-sharing basis between the partners (with consideration and consultation to what joint investment from industry could look like). 3. The horizon scanning program should establish and seek agreement on what the purpose and objectives of the horizon scanning process is (what is the research question?), how the information will be used/translated into action? (including explicit scope, audience, purpose, process/methods and outcomes/outputs). 4. The developed horizon scanning should be tied to actions required to be undertaken by the partners to prepare for the funding and successful implementation of the identified health technology. 5. A method to measure and evaluate the success of the horizon scanning program, its outputs and impacts, should be developed, and the program be regularly reviewed and updated accordingly. Continue to progress multi-agency, international collaboration around horizon scanning: Noting the international collaboration efforts the Department is already progressing, investigate if/where the information available through international collaboration on horizon scanning would meet the informational needs (or part there-of) for the purposes of the above.
Horizon Scanning to meet priority areas (including addressing equity and HUCN)	1. Establish an active horizon scanning process that to identify therapies with promising added therapeutic value, in a priority area (patient indication); This should include new therapies or new patient indications for the 'repurposing' of existing therapies. 2. This process should be open to the use of patient and clinician community partnerships, to help identify possible therapies / expanded indications, and involve them in the later parts of the process to ensure they can be informed about potential future health technologies. 3. In line with the priority reforms under the National Closing the Gap Agreement 2020 between all Governments and the Coalition of Peaks, this process should also include collaboration with ACCHS to help identify therapies for addressing areas of unmet clinical need for First Nations peoples.

	4. Develop a framework that includes an assessment of prioritisation of therapies after they have been identified through the scanning process to assist in informing the decision / action related to the identified therapy. *(note: areas of action from this proposed horizon scanning program are discussed under the section on "proactively addressing gaps* in *the PBS" and broader pathways sections)*
Horizon Scanning to help operational and capacity planning for HTA and health systems	1. Develop a method to measure and evaluate the success of the horizon scanning mechanism outlined in section 6 of the Strategic Agreement in meeting its objectives as agreed in the Strategic Agreement: a. identify major therapeutic advances which may enter the regulatory or reimbursement systems (or both) over the following 18-24 months and other trends and which may represent a significant disruption in the treatment paradigm and/or require innovation in health care system planning; and b. understand the potential implications for the Commonwealth from the introduction of these advances in terms of resources, systems and processes. 2. If this mechanism is not meeting its objectives, investigate alternative mechanisms to achieve these objectives in collaboration with industry (e.g. industry could provide advanced notice to the Department and relevant stakeholders and how that information will be tied to action, or if it would be more effective to participate in an international collaboration for horizon scanning such as PharmScan used by NICE and how this may be cost recovered).

5.3. Consideration of environmental impacts in the HTA

Environmental impact reporting	Investigate of the following options in consultation with industry and other stakeholders: 1. Reporting of environmental impacts, starting with embodied greenhouse gas emissions, in the assessment of cost-effectiveness by Australian HTA bodies. 2. Potential for use of these data in approval and reimbursement decisions. 3. Potential for public reporting of these data, to inform clinical decision-making. 4. Development of guidance documents and examples to facilitate environmental impacts reporting. 5. Alignment with international best practice in comparable jurisdictions. 6. The role of international standards for carbon foot printing of health technology products.

5.4. Mechanisms for continuous review and improvement

A program of continuous review and improvement for current HTA	This program should: 1. Be informed by consultation of internal and external stakeholders as well as research of international and interjurisdictional best practise to pick topics for review.

policies and methods	2. Have a transparent forward schedule of the consultation and planned elements and features for review. 3. Have a set time period for the reviews to be carried out (e.g. 12 months for the review of each topic or set of topics). 4. Include guidance such as the PBAC guidelines. Consideration should be given to the development of the guidance as 'living guidelines', which may be continuously updated with the evolution of new technologies and methodologies.

5.5. Capacity and capability of the HTA system

Improve HTA capacity and workforce in Australia	Develop a sponsored internship program where universities offering HTA courses and with HTA Evaluation Groups identify students for formal training in coursework. Students then undertake paid internships with the Evaluation Group to conduct evaluations, with Governments (Commonwealth and/or State/territory) to understand technology appraisal by the HTA Committee/s and policy areas, and industry (where secondment positions available). Development would be based and tracked on the HTA competencies previously developed for Government.

5.6. Strengthen international partnerships and work-sharing

A note on international Harmonisation and Work-sharing options:
The following options are designed to improve international consistency, time to listing, and HTA capacity. However, it should be noted that resource will be required for the establishment of and operation of international work-sharing pathways, and in some cases the coordination requirements for joint submissions may not result in lower resourcing requirements at the local level

Harmonisation of HTA evaluations	1. Methodology - The Commonwealth progress inter-agency collaboration and design relating to common HTA evaluation methodology, to facilitate testing and (prospective) formal introduction of HTA evaluation work sharing pathways across participating jurisdictions. 2. Timing of discussions - The Commonwealth to update its parallel scientific advice/early dialogue policies to facilitate discussions with industry sponsors, health technology users (principally clinicians and patients) and HTA and regulatory entities earlier than current arrangements (both locally or regionally where a joint evaluation is under consideration).
Work sharing for individual submissions	The Commonwealth to progress reforms to pilot work sharing pathways for individual (medicines and advanced therapies reimbursement submissions submitted across jurisdictions with comparable approaches to HTA evaluation, with a view to evaluating the merits of collaborative evaluation for reimbursement-related purposes and (if positive) embedding into the HTA framework. Available pathways should include at least one of the following options: • "Work Sharing Initiative" pathway, where concurrent reimbursement submissions are lodged in multiple jurisdictions and dossier modules are work-split amongst participating agencies

	• "Comparable Overseas Agency" (COA) pathway, where finalised HTA evaluations from comparable agencies are provided for review (with redactions for localised pricing information as strictly necessary) • Joint "Expression of Interest" (EOI) HTA pathway, where sponsors are invited by HTA agencies to bring forward priority submissions for joint reimbursement evaluation (e.g. specific rare disease treatments or treatments for narrow indications of relevance) • hybrid "sequential lodgement pathway", where dossiers may not be lodged concurrently, but access to interim evaluations from HTA agencies that are further along in HTA considerations are shared with the agreement of the sponsor to facilitate expedited local evaluation.
Collaboration with international jurisdictions to deliver sustainable access to health technologies	Investigate opportunities for collaboration with international jurisdictions to increase market share and purchasing power for innovative health technologies which address areas of HUCN.

Section 5 – Overall summary

There is broad encouragement across the submissions for the options presented in 5.1 through to 5.5. Most stakeholder groups welcomed a more proactive approach to identifying therapies that address unmet needs, horizon scanning and strong support is evident for increased environmental consideration. There were some concerns highlighted by Pharmaceutical / Medical Technology Companies about the options in 5.6 and they believed these options, such as international buying blocks, would certainly not address the issues identified.

Stakeholder groups were generally very supportive of a proactive approach to addressing unmet clinical need, developing a priority list, early engagement on the PICO and the options outlined for horizon scanning - many seeing this as one of the highest priorities. There were a number of comments about the need for widespread engagement and transparency on the development of priority list for HUCN and in horizon scanning activities. The need for clarity was raised on how the priority list would be selected and what diseases or conditions would qualify, particularly as there would be varying and competing priorities across consumer and patient groups. There was also a concern raised in regard to the heavy reliance on sponsor led submissions.

Overall, there was a very welcome response to the potential greater inclusion of environmental impacts being considered in the HTA process. A number of patient representative groups, peak bodies, clinicians and researchers highlight the impact that the healthcare system has on climate change and the environment. It is also mentioned that climate change and increased pollution have a significant impact on the health and wellbeing of patients and consumers (with asthma sufferers put forward as a key example).

In futureproofing this system, many submissions focus on the need to consider environmental impacts through all stages of HTA processes. There was discussion about environmental impacts being reported throughout assessments and particularly as part of the cost- effectiveness considerations.

There was strong support amongst stakeholders for the suggestions in 5.4, particularly around transparency and improved forward planning of consultation and review. Many submissions mentioned continuous review and improvement as pivotal to the long-term success of the HTA and to constantly be able to meet the needs of a rapidly evolving and technology driven system. As technologies and treatments change and innovate, the pharmaceutical and research stakeholders emphasised the importance of the system having adequate flexibility to accommodate assessment of these new technologies and explicit KPIs to track the success of any new reforms.

Throughout the written submissions, across a number of responses to options, the capacity, capability and resourcing of the HTA system was mentioned. There were concerns raised in regard to the capacity of the HTA committees if streamlining were to be agreed upon and implemented and there have been concerns raised about resourcing and capacity for horizon scanning to be introduced effectively and systematically. This meant there was general support for a review and overhaul of resourcing of the HTA system.

In some instances, stakeholder groups agreed that there were benefits from international partnerships and work sharing, but there were particular topics where groups highlighted some concerns. The pharmaceutical companies did not endorse or see the benefit of international purchasing or buying groups and there was a call generally across stakeholder groups for much more detail and consultation on these options.

5.1. Proactively addressing areas of unmet clinical need and gaps in the PBS

Table 76. 5.1. Proactively addressing areas of unmet clinical need and gaps in the PBS: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	9%	68%	23%	0%	0%	22
Pharmaceutical / Medical technology company	0%	38%	56%	0%	6%	16
University or research sector	0%	50%	0%	50%	0%	2
Industry association / Peak body	0%	75%	25%	0%	0%	8
Clinician (or representative organisation)	0%	100%	0%	0%	0%	2
Consulting	0%	0%	100%	0%	0%	1
State / Territory government	0%	0%	100%	0%	0%	2
Other	0%	50%	17%	33%	0%	6

Patients, Consumers and Representative Groups

These stakeholder groups were generally very supportive of a proactive approach to addressing unmet clinical need and the current gaps in the PBS. There were a number of comments about the need for widespread engagement on the development of priority list for HUCN, and the need for clarity as to how the list would be selected and what diseases or conditions would qualify, particularly as there would be varying and competing priorities across consumer and patient groups. There was concern raised in regard to the heavy reliance on sponsor led submissions.

"We are very supportive of a more proactive approach to identifying unmet needs and potential therapies that may address them, as well as processes that provide a path for such therapies to be made available to Australian patients." (PRIMCAT Consumer Panel - Independent Consumer Panel)

"Proactively identifying unmet need, horizon scanning and proactively inviting and incentivising submissions would make a huge difference to the childhood dementia community." (Childhood Dementia Initiative)

"Again, clear criteria is needed to define who should be included in a priority list. There should be strong consultation across the sector for how to decide on the priority list." (MND Australia)

"As stated earlier, NAA members are particularly interested in clarity and equity in how areas of high unmet clinical need (HUCN) are defined in the implementation of these reforms. There are many rare diseases among NAA members, with no or limited treatment options/therapeutics, and there is a risk that the new HUCN criteria will prioritise larger cohorts." (Neurological Alliance Australia)

"It is critical to prioritise the future of healthcare. In many areas, Australia lags behind other first-world nations. However, the reliance on a sponsor is too heavy. There is significant clinical research taking place independently which could lead to better health outcomes for Australians. A cross section of data must be considered and not simply be sponsor led/dependent. Further, international collaboration should be considered to further these objectives." (Cystic Fibrosis Australia)

"*Unmet clinical need is a difficult area to assess given the many and varied competing priorities. Any priority list must be developed based on a clearly established and agreed set of criteria that accurately reflect how unmet need is viewed - by consumers, by clinicians, by industry, by organisations including not for profits and by the government. Horizon scanning must be an intrinsic mechanism built into the work of the HTA NOT an afterthought. How the horizon scanning is conducted and what it identifies needs to be clearly presented in a timely manner so that all stakeholders are aware of potential promising therapies. How promising therapies are assessed and prioritised must be transparently and clearly communicated with easy to understand information relating to decision making processes. Once a promising therapy has been identified and assessed supporting its prioritisation through additional incentives would be supported ONLY if the clinical evidence supports the use of such therapies. Consideration also needs to be given to the application of promising therapies to more than one health priority area*
- confining a therapy that has multiple applications of impact and benefit across multiple health conditions must form part of assessment processes." (Anonymous submission)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies were predominantly positive about these options but warned of the potential risks of the priority list if not carefully planned, implemented and managed, they requested more clarity on this. They also questioned how the development of the list would be equitable for all of the varying and competing priorities. One of these companies identified an opportunity to design a subset of this list with First Nations people specifically.

"The Options paper notes that stakeholders suggest a more proactive approach to identifying therapies that address unmet needs in Australia. The paper suggests that development of a priority list of areas of unmet need and horizon scanning be undertaken to better prepare the Australian health system. AZ agree these options would have a positive impact" (AstraZeneca).

"Alexion supports this approach in principle. A priority list should not exclude rare diseases in its consideration and should be equitable for all patients and disease areas. However, a priority list should avoid any movement towards a Pharmac-style system which has severely limited access to therapies for New Zealanders. An IQVIA study (September 2023) found that there are 131

modern medicines available through public funding for Australian patients that are not available to New Zealand patients." (Alexion)

"This proposal needs to be planned and implemented carefully to avoid an overly restrictive priority list, with a clear framework to ensure that all stakeholders know what to expect. There should be no artificial limits on disease areas, population sizes or technology types. Adequate and early stakeholder consultation including industry on creation of the priority list and horizon scanning will be essential. Early PICO scoping should be an interactive and consultative process that is also pragmatic in terms of the resources required by all stakeholders. This proposal could be effective but must be guided by clinical need rather than budget considerations and would need to be subject to regular review to ensure the priorities remain current. The option for proactive submission invitation and incentivisation includes impractical timelines and must be further consulted to ensure any new approach can actually work in practice to deliver for all stakeholders." (Pfizer)

"Currently, some Sponsors already proactively submit drugs for early assessment. If there is proactivity from the Department of Health and Aged Care, a clearer framework would be crucial to manage expectations. The proposed 4-6 week timeframe for accepting these invitations to apply is an unnecessary restriction and in practice, it would be almost impossible to achieve due to internal approval and governance processes. is impractical for global companies. A longer timeframe is needed in order to ensure an appropriate assessment of the risks, benefits, and uncertainties involved, especially for repurposed medicines or other technologies with limited evidence. To incentivize participation, considering exemptions from standard price reductions or other attractive benefits could be key. Overall, it is also unclear how the priorities would be determined." (UCB Australia)

"Roche supports the development of a priority list of high unmet clinical need priority areas and the potential future opportunity to accelerate access for treatments in these areas. Roche notes that the development of a priority list should not come at the expense of established pathways and consideration of technologies that may not address an area of high unmet clinical need (i.e. a therapy that may have high added therapeutic value, but not in a HUCN). Further detail and clarity are needed on how this list would compare and connect with National Health Priority areas. Likewise for antimicrobials and vaccines, further clarity would be required to understand the rationale for any significant non-regional specific deviation from the WHO's Global Priority Pathogens or Vaccine-Preventable Diseases lists. Consistent with Option 1.3, Roche also supports a subset of the priority list to be developed in partnership with First Nations people representative organisations for areas of unmet clinical need and gaps in funded access for First Nations peoples." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was support from these stakeholder groups with some commentary about the need for flexibility and the recommendation of a rating system for HUCN.

"I support this initiative. However, I wonder whether the kind of companies provided fee waiver should be considered. Should this be means tested in some way? Should incentivization be linked to the urgency of the situation? Throughout all of these processes ensuring that the carbon emissions and waste associated with these products should happen up front." (The University of Notre Dame Australia)

"Support in principle this proposal because the system needs to be flexible and combining with horizon scanning use all of the opportunity to get value for money. Important to use infrastructure that already exists. Transparency and consultation." (Immunisation Coalition)

"'Unmet needs'" must be defined and a rating must be allocated. It will be seen differently according to the aetiology and difficulty of treating a disease and the familiarity/understanding of the members of any committee/team making decisions. Prioritisation is likely to be biased and inequitable simply due to lack of specialist knowledge. There needs to be exceptional transparency on how prioritisation is decided as well as a public list of medicines being considered and the queue in order of priority of the others that will be evaluated." (Consumer and independent researcher)

"This has been written in a way that is only focused on the PBS. This needs to be expanded to all technologies. The responses below assume it is extended to MedTech and digital health technologies" (Medical Technology Association of Australia)

Table 77. Development of a priority list – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	5%	30%	60%	5%	20
Pharmaceutical / Medical technology company	0%	6%	24%	59%	0%	12%	17
University or research sector	0%	0%	33%	0%	67%	0%	3
Industry association / Peak body	0%	0%	0%	57%	43%	0%	7
Clinician (or representative organisation)	0%	0%	50%	0%	50%	0%	2
Consulting	0%	0%	0%	100%	0%	0%	1
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	0%	20%	40%	40%	0%	5

Patients, Consumers and Representative Groups

This option is well supported by patient and consumer representative groups with the suggestion that it should be developed transparently and with the voice of the consumer included. There are also concerns about how the conditions on this list will be selected. More detail around this option has been requested. Adding children to the priority list was seen by these groups as an opportunity to incentivise sponsors to bring new paediatric medicines to market, if suitable medicines can be found through the proposed horizon-scanning capability.

"Painaustralia supports measures to proactively address areas of unmet clinical need and gaps in the PBS (5.1). Painaustralia concurs that any such measures would 'require methodological development, implementation planning, and adequate resourcing including joint investment across stakeholder groups. It is Painaustralia's view that the development of a priority list of high

unmet clinical need; identification of therapies to meet priority lists; and early assessment and prioritisation of potentially promising therapies would assist in this regard." (Painaustralia)

"It will be important to ensure that the process of developing the priorities is transparent and that consumer voice is present here. The process must not be subject to power imbalance where industry or well-funded consumer groups dominate the priorities established." PRIMCAT Consumer Panel (Independent Consumer Panel)

"*We would like to see children be listed as a priority group. There is a lack of medicines for children in Australia, including asthma medicines, when compared with countries with similar economies."* (Asthma Australia)

"CHF supports the development of a priority list for high unmet clinical need, developed in partnership between clinicians, patient organisations, and community. CHF also welcomes the development of priority areas in partnership with Aboriginal Community Controlled Organisations (ACCOs)." (Consumers Health Forum of Australia)

"Essential the priority list development explicitly involves consumers, consumer organisations and clinician experts from the outset." (NeuroEndocrine Cancer Australia)

"More detail is required here." (Anonymous submission)

"More information is need on how a priority list will be compiled. There are 145 rare genetic disorders that cause dementia in childhood and have no treatments. Will they all be on the list?" (Childhood Dementia Initiative)

Pharmaceutical / Medical Technology Companies

There was broad support for the development of a priority list and commentary on how it would be developed, with further industry engagement commonly requested. There were some concerns raised that this list should not result in the deprioritisation of other areas.

"We strongly support the inclusion of surveillance of AMR and vaccine preventable diseases in the priority list." (GSK)

"Industry should be involved in the development of a priority list for HUCN." (Novartis Australia)

"Supportive of prioritisation of therapies representing important advances in areas of HCUN, but not if it results in the active deprioritisation of other medicines." (Eli Lilly Australia)

"Prioritization around unmet clinical need and gaps in the PBS must not result in other areas being deprioritized for review." (Bayer Pharmaceuticals ANZ)

"As is observed with other countries (i.e. NZ), a priority list for funding is somewhat redundant if there are no policies to support innovative medicines. The list continues to grow and the notion of a treatment being a priority is arguably a futile exercise. Any prioritisation around unmet clinical need and gaps in the PBS must not result in other areas being deprioritised (such as rare disease treatments)." (Biogen)

"(AbbVie's position is that a priority list should be created and maintained for the purpose of forward-focused horizon scanning only, and not be related to any value and/or funding decisions. This would present an unacceptable level of risk for Sponsors and patients, particularly if this led to the deprioritisation of what would be considered "non-priority" treatments. It is vitally important that there is sufficient capacity within the HTA evaluation process to adopt an "and" not an "or" approach to the consideration of submissions." (AbbVie)

"Roche supports the development of a priority list of high unmet clinical need priority areas and the potential future opportunity to accelerate access for treatments in these areas. Roche notes that the development of a priority list should not come at the expense of established pathways for technologies that may not address an area of high unmet clinical need (i.e.. a therapy that may have high added therapeutic value, but not in a HUCN). Roche notes that focussing solely in areas of priority may result in the unintended consequence of therapeutic advances, be it therapies that have high added therapeutic value but not in an area of HUCN, or add value to patients or healthcare systems that may not have a demonstrable improvement in health outcomes, becoming less attractive to launch in Australia, impacting patient and clinician choice." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was broad support for this option with these stakeholder groups, they did highlight however the need for a diverse group of stakeholders to be consulted on the development of the list, with stakeholders again highlighting that there could not be any deprioritisation of other areas.

"The development of a priority list for HUCN, informed by diverse stakeholders and inclusive of specific considerations for Indigenous health and public health surveillance, strengthens the HTA process and ensures that healthcare resources are directed toward areas of greatest need." (Society of Hospital Pharmacists of Australia)

"Seems entirely sensible. It will be important that the priority list has sufficient detail and specification so that it has meaning." (THEMA Consulting)

"In general, I would support a more proactive system that seeks future opportunities as long as it is adequately resourced and informed by experts with the aim of bringing new therapies to patients in a more timely manner." (Clinician)

Table 78. Identifying therapies to meet priority list (horizon scanning) – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	5%	33%	62%	0%	21
Pharmaceutical / Medical technology company	0%	0%	38%	56%	0%	6%	16
University or research sector	0%	0%	33%	0%	67%	0%	3
Industry association / Peak body	0%	0%	0%	50%	50%	0%	8
Clinician (or representative organisation)	0%	0%	0%	50%	50%	0%	2
Consulting	0%	0%	100%	0%	0%	0%	1
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	0%	20%	20%	60%	0%	5

Patients, Consumers and Representative Groups

There was a great deal of support for horizon scanning, many called it out as a very high priority for improving the HTA system and requested both international and consumer engagement and collaboration from the outset.

The Collaborative Consumer Group Response submission emphasised the need to *"ensure horizon scanning and prioritisation approaches are codesigned and mandate consumer involvement in horizon scanning and prioritisation activities. It is not clear how community perspectives and priorities will be identified and these should inform any horizon scanning and prioritisation activities."* (Collaborative Consumer Group Response)

"Formal HTA approaches must identify and accommodate major therapeutic advances for the treatment and management of chronic pain that may enter the regulatory or reimbursement systems (or both). The contemporary evidence base underpinning therapeutic innovations for pain management supports the use of therapies that include consideration of the pain experience from a biomedical and biopsychosocial perspective. This includes both pharmacological and nonpharmacological therapies." (Painaustralia)

"MSCAN welcomes the focus in the Options paper on Horizon Scanning. This is an incredibly important component of improving the HTA process and delivering better outcomes for Australia's patients. Workshops that facilitate multistakeholder consultation should be at the core of a horizon scanning system to ensure needs, gaps, possible changes and preparedness is aligned and well considered." (Melanoma & Skin Cancer Advocacy Network (MSCAN))

"Essential that horizon scanning explicitly involves consumers, consumer organisations and clinician experts from the outset." (NeuroEndocrine Cancer Australia)

"Patient organisations often have detailed information about therapies on the horizon that is not easily obtained the public domain. Partnerships with patient organisations in this horizon scanning would increase efficiency and accuracy." (Childhood Dementia Initiative)

"CHF welcomes and supports the development of horizon scanning capacities in Australia, as well as direct mention of a partnership mechanism with Aboriginal Community Controlled Organisations (ACCOs) to ensure health outcomes and equity for First Nations Peoples are prioritised. The options mention that horizon scanning should be "open" to the use of patient and clinician partnership. CHF argues that a stronger commitment to consumer involvement is necessary to ensure that the activities of horizon scanning bodies reflect the real needs of the community." (Consumers Health Forum of Australia)

"More detail is required here and international collaboration is essential." (Cystic Fibrosis Australia

Pharmaceutical / Medical Technology Companies

These companies broadly supported horizon scanning and believed that it was crucial in order to adopt a truly proactive and forward focused approach.

"Industry should be involved in the development of a priority list for HUCN." (Novartis Australia)

"Using horizon scanning to identify therapies to meet the priority list is appropriate, if a fit-for-purpose horizon scanning system is established." (Eli Lilly Australia)

"The potential for company-specific pipeline discussions should be explored in order to truly adopt a forward-focused approach to understanding future health technologies and to support operational and capacity planning. AbbVie would welcome the opportunity to enter into an open dialogue at regular cadence with the PBAC and DoHAC on pipeline technologies." (AbbVie)

"Repurposing of medicines naturally fits with industry principles and the NMP principle of expanding access in a sustainable way. Repurposing medicines can be complex, and hence requires opportunism from all stakeholders (regulators, industry, payers, clinicians and patients). Previous consultations that have discussed options that include compelling sponsor to submit applications, or allow non-sponsor applications (i.e. clinicians, other organisations) fail to recognise the supply chain and demand planning considerations for non-sponsor submission. The relaxing of other pricing (price disclosure, SPRs, SPAs) and cost recovery (fee waivers) polices should be looked at as incentives for repurposing." (Biogen)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was strong support for horizon scanning amongst these groups, there was also a few additional suggestions for consideration - these have been highlighted below.

"Strongly supported, this may also give a lot of credibility to investigator research that is underway or planned and bolster its relevance in the determination of the HTA." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

"By systematically scanning for new therapies or new indications for existing therapies, this approach ensures that emerging healthcare innovations are promptly evaluated and considered for funding. Partnering with ACCHSs to ensure the inclusion of First Nations peoples' health outcomes and health equity in the horizon scanning process is essential for addressing disparities in healthcare access and outcomes. This partnership approach recognises the importance of Indigenous perspectives and priorities in identifying therapies to meet the needs of diverse patient populations. Furthermore, including technologies without market authorisation in Australia and exploring opportunities for repurposing existing therapies for new indications broadens the scope of the horizon scanning process. This comprehensive approach maximises the potential to identify innovative solutions and address gaps in funded access across various healthcare settings." (Society of Hospital Pharmacists of Australia)

Table 79. Early assessment and prioritisation of potentially promising therapies – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	5%	20%	75%	0%	20
Pharmaceutical / Medical technology company	0%	0%	27%	60%	0%	13%	15
University or research sector	0%	0%	33%	33%	33%	0%	3
Industry association / Peak body	0%	0%	0%	57%	43%	0%	7
Clinician (or representative organisation)	0%	0%	0%	50%	50%	0%	2
Consulting	0%	0%	0%	100%	0%	0%	1
State / Territory government	0%	0%	50%	50%	0%	0%	2
Other	0%	0%	25%	25%	50%	0%	4

Patients, Consumers and Representative Groups

There was a very positive response to this option from consumer and patient representative groups, with only a small amount of additional commentary provided.

"With the narrow therapeutic window of progressive, fatal diseases like the childhood dementia disorders, prioritisation of breakthrough drugs could potentially save many lives." (Childhood Dementia Initiative)

"Essential that this early assessment and prioritisation explicitly involves consumers, consumer organisations and clinician experts from the outset." (NeuroEndocrine Cancer Australia)

Pharmaceutical / Medical Technology Companies

Overall, there was also a very positive response from industry in regard to this option and it was seen to have a number of potential positive impacts, but more information was requested.

"Assessment of whether a therapy is potentially promising, to enable prioritisation, can have positive impacts. Understanding the criteria of the potential promise requires further clarity. Early attempts at HTA assessment are not appropriate as there is insufficient information at that stage to draw meaningful conclusions." (Eli Lilly Australia)

"Roche would support this option if the outcome resulted in the prioritisation of therapies that would represent important advances in areas of HUCN. However, more detail on this option is required to make a full assessment of its ability to address the issues outlined. This detail should include how these particular therapies will be identified through a pipeline and directed through a prioritised process. There is also the possibility of an unintended consequence of this option leading to the de-prioritisation of other therapies with similar health benefits launching in Australia, as they will be ineligible for this pathway." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Further support and encouragement were received from these groups, but more detail was requested. There was also a warning from one stakeholder about the risks associated with assessing a specific health technology too early without access to the best available evidence.

"By engaging in early assessment and prioritisation, healthcare decision-makers can make informed choices about which therapies to prioritise for further evaluation and potential funding. This systematic approach enhances the effectiveness of the HTA process by focusing attention and resources on therapies that have the most significant impact on addressing unmet clinical needs and improving patient care." (Society of Hospital Pharmacists of Australia)

"Seems reasonable. However, I caution that if this assessment is done too early without access to all the best available evidence there could be the potential to unfairly de-prioritise and harm the reputation of a given intervention." (THEMA Consulting)

"On face value this seems reasonable as long as the process does not slow down access to medications overall..." (Clinician)

Table 80. Proactive submission invitation and incentivisation – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	5%	20%	70%	5%	20
Pharmaceutical / Medical technology company	0%	0%	38%	44%	0%	19%	16
University or research sector	0%	0%	67%	33%	0%	0%	3
Industry association / Peak body	0%	0%	14%	43%	43%	0%	7
Clinician (or representative organisation)	0%	0%	0%	50%	50%	0%	2
Consulting	0%	0%	0%	100%	0%	0%	1

State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	0%	25%	25%	25%	25%	4

Patients, Consumers and Representative Groups

This option again received very positive feedback from these groups, there were a number of potential positive opportunities identified by these groups including the possibility that as a result of this option being introduced, it could potentially incentivise sponsors to put forward submissions for smaller populations. One further suggestion was that the role of non-commercial sponsorship should be explicitly acknowledged.

"There are treatments for some of these rare diseases approved overseas but the companies are not even considering submitting an application in Australia because the population is too small and they are small companies, often not familiar with Australian processes. Examples of this are gene therapies for adrenoleukodystrophy and metachromatic leukodystrophy." – (Childhood Dementia Initiative)

"Once a therapy is identified through horizon scanning and has been prioritised through the early assessment, the Government could proactively request a sponsor submission with incentives for a sponsor to bring this forward." (NeuroEndocrine Cancer Australia)

"This option still focuses on sponsor submissions. The role of non-commercial sponsorship should be explicitly acknowledged and work on this pathway should be part of this option. This should include submissions by non-government organisations and professional bodies." (Mito Foundation)

Pharmaceutical / Medical Technology Companies

Industry expressed support for this option and believed incentives and invitations had the potential for positive impacts. There were some suggestions that a framework was needed and that incentives for early reimbursement or repurposing need to be long term for a sustainable listing.

"Submission invitation and incentivisation can have a positive impact, but 4-6 weeks for notification of acceptance is insufficient time. 2-3 months would be more appropriate." (Eli Lilly Australia)

"This already happens from time to time. This proposal should include a framework, with a time frame longer than 4 to 6 weeks for sponsors to respond to the invitation; this time frame does not allow sponsors like Menarini sufficient time to assess the risks and benefits to the company or find licencing partners if needed. Further, sponsors should be incentivised to accept, for example, through exemptions from the standard price reductions, and exclusivity arrangements." (A.Menarini Australia)

"Incentives for early reimbursement applications or for repurposing products need to be long term for sustainable listing. the Review document describes some incentives such as provision of a case worker and cost-recovery fee exemptions but the costs of listing either new therapies early or repurposing other products would require different types of incentives. For example,

potential incentives to encourage an early reimbursement submission could include a greater willingness to accept clinical and economic uncertainty when evaluating the therapy, complete confidential pricing for the period that the therapies clinical data is considered immature, or no budget expenditure caps. For products that are repurposed exemptions from price referencing, lowest cost comparator and impact on the other indications of the product would be required." (Novartis Australia)

"Proactive requests for sponsor submissions already occur. It would be helpful to design a framework so that both parties (government and sponsor) know what to expect. The proposed timeframe for offer acceptance is unrealistic to gain global endorsement and to conduct necessary assessments of risk and benefits. These medicines may have uncertainty in their evidence, consideration of incentives for companies to make the proposition viable; for example, fee-wavering, or exemptions from standard price reductions may need to be introduced to make this option workable." (Bayer Pharmaceuticals ANZ)

"Roche supports the option of incentives to encourage prioritising therapies identified through horizon scanning. The outlined options may help to address the current issues with attracting therapies which address areas of HUCN, however, Roche does not believe that these incentives replace appropriate value recognition commensurate with a technology that addresses HUCN, to ensure Australia remains attractive as a first launch country. Similarly, a provisional funding program for patients to obtain access would also only be viable if an acceptable pricing arrangement could be agreed to by sponsors and the Government. Timelines also need to be jointly agreed with the sponsor rather than a pre-defined notification to Government with the acceptance of a proactive submission offer. Depending on the situation and circumstance, a 4-6 week time period may be insufficient to assess the potential viability and consequences, within and external to Australia." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These groups were positive and supportive of this option, they did however offer a few individual suggestions that have been included below.

"Supported, conflict of interests must be declared and it will be important that selection bias does not occur." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

"While the process issues/fees would be welcome, I think it would also be important that interventions for these priority areas are afforded special decision-making consideration also. EG: all else equal a treatment in a priority area would attract a higher cost/QALY threshold. this could easily be accommodated in the explicit value framework." (THEMA Consulting)

"Fee waivers for any submissions for repurposing drugs at the end of their patent or off patent are essential." (Consumer and independent researcher)

Table 81. Early PICO scoping – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	40%	50%	10%	20
Pharmaceutical / Medical technology company	0%	0%	20%	53%	13%	13%	15
University or research sector	0%	0%	33%	67%	0%	0%	3
Industry association / Peak body	0%	0%	0%	100%	0%	0%	7
Clinician (or representative organisation)	0%	0%	0%	50%	50%	0%	2
Consulting	0%	0%	0%	100%	0%	0%	1
State / Territory government							0
Other	0%	0%	33%	33%	0%	33%	3

Patients, Consumers and Representative Groups

There was a very positive response to this option, with a comment highlighting the need for consumer engagement at the earliest possible stage in the process.

"Post early horizon scanning, absolutely non-negotiable that this process must involve consumers, consumer organisations and clinician stakeholders from the earliest stage of PICO development." (NeuroEndocrine Cancer Australia)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies were generally supportive of this option and believed that early alignment on the PICO could reduce delays, early adoption by clinicians and allow potential stakeholders to prepare for the introductions of the new technology.

"Aligning on the PICO early would ensure that there are no delays in recommendation due to different expectations of the PICO and could be beneficial to all medicines in the future." (Eli Lilly Australia)

"Roche supports a PICO scoping phase, especially in the circumstances where implementation requirements and challenges can be identified. Roche notes that early PICO scoping would be particularly useful for the preparation of potential stakeholders impacted by the introduction of a new technology. From an industry perspective, early PICO scoping would be useful to ensure more rapid adoption of the technology within the clinical community, once funded, ensuring that the benefit from the technology can be optimised as soon as it becomes available." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These groups supported early PICO engagement and believed it was a sensible approach that could aid efficiencies and effectiveness in the HTA process.

"Early PICO scoping enhances the efficiency and effectiveness of the HTA process by ensuring that key evaluation parameters are identified and addressed early on." (Society of Hospital Pharmacists of Australia)

"Contemporaneously with the development of the submission itself makes sense." (THEMA Consulting)

5.2. Establishment of horizon scanning programs to address specific informational needs within HTA and the health system

Table 82. 5.2. Establishment of horizon scanning programs to address specific informational needs within HTA and the health system into account: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	5%	79%	11%	0%	5%	19
Pharmaceutical / Medical technology company	0%	63%	31%	6%	0%	16
University or research sector	25%	25%	25%	0%	25%	4
Industry association / Peak body	0%	75%	25%	0%	0%	8
Clinician (or representative organisation)	0%	100%	0%	0%	0%	2
Consulting	0%	100%	0%	0%	0%	1
State / Territory government	0%	0%	100%	0%	0%	2
Other	0%	75%	25%	0%	0%	4

Patients, Consumers and Representative Groups

There was strong support identified from stakeholders in these groups. Many advocated for consumer engagement in these programs and emphasised horizon scanning programs are critical, particularly for those with rare diseases.

"Horizon scanning to facilitate timely planning and adoption ahead of TGA sponsor applications is crucial in these times of rapidly evolving therapeutic technologies. It is also important to ensure that there is a disease-specific as well as a broader approach to this in stakeholder input and opportunities for each group to learn from each other. This option has the potential to build on existing strengths in the blood products area and National Blood Arrangements. With low patient numbers worldwide, rare diseases are by their nature not conducive to large national clinical

studies. International networks in the disease area are often already active in collaborating, sharing data to aggregate larger data results and developing best practice clinical guidelines. They also collaborate to develop and validate health-condition specific benchmarks and evaluation tools." (Haemophilia Foundation Australia)

"NAA members are excited about the prospect of horizon scanning to both address inequity and support timely access and look forward to further details on how this process will be implemented, including the evaluations process." (Neurological Alliance Australia)

"Essential that consumers informational needs are addressed with this change- Patients are not treated equitably, as information for options to alternate access e.g. TGA special access scheme, compassionate access or the Medical Treatment Overseas Plan is complex, fragmented and is very poorly understood by most health care providers." (NeuroEndocrine Cancer Australia)

"Definitely need to include all stakeholders including patients/consumers and support organisations." (Genetic Support Network of Victoria)

"Lung Foundation Australia strongly supports horizon scanning. We note that consumer engagement/involvement in this process is not fully developed. We advocate for consumer engagement being incorporated into the horizon scanning process. For horizon scanning to work, and work effectively and efficiently, it must:

- *be forward facing and future proof with a minimum of five years to decrease lag time to clinical application and approval as it presently is impacting too many Australians;*
- *be enhanced to ensure that Australians are provided with timely access to new drugs and novel medical technologies, including for rare diseases; and,*
- *include Patient Reported Outcome Measures (PROMs) and Patient Reported Experience Measures (PREMs) as the absence of these across the continuum limits the quality of care and value of health services being delivered."* (Lung Foundation Australia)

"Proactive horizon scanning is very important for rare diseases. For rare diseases especially, the process should focus on:

- *Earlier and better consumer involvement, including partnerships with consumers and clinicians to participate in horizon scanning,*
- *Support for these partnerships, especially for individual consumers/smaller consumer organisations,*
- *Transparency in processes and criteria for HUCN."* (Mito Foundation)

"The solutions proposed appear to lack avenues through which consumers can actively engage in this horizon scanning process. Once again, and based on initial comments, consumers should be involved in every aspect of assessment particularly when discussing programs to address specific information needs within the HTA and the health system. Further work needs to be done on this solution to better articulate how consumers and consumer focussed organisations (not for profits) will be actively engaged as relevant stakeholders to support forward planning and

priority setting. Consumers, particularly those with high health literacy, are often the people who have advanced knowledge of disruptive and advanced therapies. The HTA must seek every opportunity to allow for consumer engagement and contribution to identifying and prioritising these therapies." (Anonymous submission)

"Who undertakes the Horizon Scanning? And what are the criteria? Again, a clear definition for HUCN is needed." (MND Australia)

Pharmaceutical / Medical Technology Companies

There was broad support from companies, however many highlighted the need for significant consultation and more detail in regard to these programs, as well as the need for international collaboration.

"Horizon scanning offers the greatest benefit when it enables meaningful preparation and action from impacted stakeholders. Roche supports the level of consultation described in the Options paper; engaging with relevant Committees, Commonwealth, State and Territory Governments, and industry. Significant consultation would be required to understand expectations of the joint investment from industry to warrant the support in horizon scanning. Roche notes that to optimise the introduction of horizon scanning, international collaborations where extensive investment has already gone into establishing horizon scanning processes should be leveraged wherever possible. Roche recommends that the current aim of 'addressing specific informational needs' is a first step in ensuring a responsive HTA and health policy system is prepared and ready to enable the delivery of new healthcare innovations." (Roche Products)

"We support the implementation of horizon scanning programs." (UCB Australia)

"This proposal is reasonable but needs to be implemented carefully to avoid an overly restrictive priority list as discussed in our response to Option 5.1. A stronger justification and clearer model for cost sharing with industry should be provided. There should be a clearly defined role of horizon scanning aligned with section 6.2.1 of the Strategic Agreement to promote greater understanding and insight into new and emerging technologies to facilitate faster access for Australian patients. As with the proposal above, any horizon scanning work should be guided by unmet clinical need not budget considerations and subject to ongoing review." (Pfizer)

"Boehringer Ingelheim supports the option for horizon scanning programs. However, it is essential that these programs are guided by well-defined and publicly available disease priorities established by the Department. Any implementation of horizon scanning programs should be accompanied by augmented investment in the PBS." (Boehringer Ingelheim)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Again, broad support was highlighted here. One stakeholder did emphasise the need to measure and evaluate the success of this program and requested information on the goals and KPIs being considered.

"AHHA supports the introduction of a horizon scanning program focused on meeting the needs of Australians." (Australian Healthcare and Hospitals Association)

"This seemed like a considered approach. I consider that all steps should be considering the carbon emissions of these products. If the carbon footprint is excessive that may be the trigger for halting the whole process." (The University of Notre Dame Australia)

"We suggest the scope for review of the impact of HTA for access to health technologies be extended to include the full spectrum of diagnostic tools and supporting technology, not just those within the narrow use of medicines. Restricting horizon scanning to technologies that impact the PBS leaves much of the impact of other IVD technologies outside the reach of our healthcare system." (Pathology Technology Australia)

Table 83. Horizon scanning for advanced therapies (including high cost, HSTs funded through the NHRA) and other potentially disruptive technologies – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	38%	56%	6%	16
Pharmaceutical / Medical technology company	0%	0%	27%	67%	7%	0%	15
University or research sector	0%	0%	50%	50%	0%	0%	4
Industry association / Peak body	0%	0%	14%	57%	29%	0%	7
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	1
Consulting	0%	0%	0%	100%	0%	0%	1
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	25%	25%	25%	25%	0%	4

Patients, Consumers and Representative Groups

There was support for horizon scanning amongst these stakeholder groups and there was hope that by horizon scanning internationally for advanced therapies that this could decrease the time it takes to access innovative therapies in Australia.

"International horizon scanning to see what therapies are advancing in the pipeline and could have benefits for Australians is standard practice in bleeding disorders. Australia has a history of slow access to innovative therapies, as has been our experience with bleeding disorders. For example, it took nearly three years from registration to funding approval in 2020 for an important innovative therapy for haemophilia A, omalizumab/Hemlibra®. This meant that Australian patients with inhibitors and severe haemophilia continued to live in pain, experience bleeding episodes, hospitalisations and poor quality of life while patients in other countries with similar health economies already had access to this therapy for some years. However, this horizon scanning and monitoring of experience in other similar countries can provide an opportunity for

valuable collaborations among stakeholders to prepare the ground for them." (Haemophilia Foundation Australia)

"We are excited about the prospect of horizon scanning to both address inequity and support timely access for patients with rare diseases and high unmet need, as is the case for childhood dementia." (Childhood Dementia Initiative)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies could see the possibilities for benefits in horizon scanning for advanced therapies, they did however request clarity and more detail on a number of factors including resourcing, responsibility and accountability, funding and joint investment. There was one potential issue/hurdle flagged in regard to how much information would be available publicly from international companies, who tend to keep their information commercial in confidence, with restricted access, until market entry.

"Positive provided the horizon scanning triggers meaningful actions and progression to submission and listings." (Johnson and Johnson Innovative Medicines)

"Horizon scanning for advanced therapies and other potentially disruptive technologies can be of great benefit, and partnership with the Commonwealth, state and territory governments and industry is imperative. However, it is the responsibility of the government to provide the infrastructure and the resource to implement an effective horizon scanning system, given that the purpose is to prepare Australian health systems for appropriate budgeting, capacity planning and implementation of new technologies. Whilst the horizon scanning system should be able to allow the identification of high-cost HST's, it should not be restricted to high cost therapies, as low cost therapies (e.g. digital therapies) may cause significant disruption that requires future planning." (Eli Lilly Australia)

"Roche supports in principle horizon scanning for advanced therapies, noting that further clarity and consultation is required in a number of areas. Roche recommends:

- *A commitment to the establishment, responsibility and accountability for horizon scanning with clear and regular timelines for meetings and reporting,*
- *An 'enduring' structure is established to ensure continuity and consistency for horizon scanning. As seen with previous structures, such as HealthPACT, horizon scanning efforts were disbanded when the Australian Health Ministers' Advisory Council (its parent committee) was dismantled;*
- *Early and meaningful industry engagement to ensure critical endorsement, noting NHRA consultation and current International HTA Collaboration meetings have not been extended to industry;*
- *Further consultation and clarity on the rationale for 'cost-sharing' and 'joint investment' from industry in the absence of agreement on the scope and objectives of the horizon scanning process; and*

- *Agreement on the proposed scope which is currently stated to include 'advanced therapies' and 'other potentially disruptive technologies'."* (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was a positive response from these stakeholder groups who suggested that with all the relevant jurisdictions and stakeholders engaged, that this would ensure a comprehensive and collaborative program.

"The structured scanning process seemed robust. Good that it included a method to measure and evaluate the success." (The University of Notre Dame Australia)

"By involving all jurisdictions and relevant stakeholders, including healthcare agencies and industry, this approach ensures comprehensive coverage and collaboration." (Society of Hospital Pharmacists of Australia)

Table 84. Horizon Scanning to meet priority areas (including addressing equity and HUCN) – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	39%	56%	6%	18
Pharmaceutical / Medical technology company	0%	0%	20%	60%	13%	7%	15
University or research sector	0%	0%	50%	25%	25%	0%	4
Industry association / Peak body	0%	0%	11%	67%	22%	0%	9
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	1
Consulting	0%	0%	0%	100%	0%	0%	1
State / Territory government	0%	0%	100%	0%	0%	0%	2
Other	0%	0%	20%	40%	40%	0%	5

Patients, Consumers and Representative Groups

This supported overwhelmingly by these groups who said they have advocated for this option for many years and who said they had been shouldering the burden of horizon scanning themselves for some time. It was suggested that specific capacity-building for rarer disease types could be included as a recommendation, as this was said to be resource-intensive.

"Our consumers and our organisation have advocated for many years for the inclusion of a systemic approach and pathway to enable faster access to therapies for underrepresented populations of patients due to a lack of horizon scanning or a commercial sponsor to make a submission." (NeuroEndocrine Cancer Australia)

"Proactive and dedicated horizon scanning process was highlighted by BCNA as a key recommendation in our original Phase 1 HTA consultation response. Currently, the burden of horizon scanning is left to patients and patient groups in a majority of incidences, placing an unfair burden on smaller and less resourced disease types and meaning Australia's HTA is seldom prepared ahead of time for novel therapies and new types of treatment options. In oncology, these include precision medicines, genomics, antibody-drug conjugates, and drugs for new breast cancer subtypes such as HER2-low. BCNA was pleased to see dedicated horizon scanning processes recommended in the Options Paper but notes that only tentative language is used to suggest the involvement of patients and patient groups who are currently central to this process and must be involved in horizon scanning processes moving forward. BCNA also notes that this is a resource-intensive process and questions whether specific capacity-building for rarer disease types could be included as a recommendation, and that processes concerned with horizon scanning are established in partnership with those already doing this work across the NFP and research sectors." (Breast Cancer Network Australia)

Pharmaceutical / Medical Technology Companies

There was support for this option, but it was emphasised that careful planning was required and consideration given to a broader focus of horizon scanning, not just through the lens of HUCN.

"A horizon scanning system that has been developed with clear objectives should be able to allow scanning to identify therapies that address priority areas. But careful planning of the system is essential." (Eli Lilly Australia)

"Roche supports horizon scanning to raise early awareness of emerging new health technologies that will impact health system policies, administration and funding in the mid to long term. Without an embedded horizon scanning model that has clear scope, objectives and process, Roche is concerned with a potentially premature narrow focus on priority areas. Whilst it is important to include topics where equity and HUCN are identified, these should be in addition to, and not at the expense of, examining information on other health technologies and disruptive therapies.

It is unclear how horizon scanning focused on HUCN or priority populations will further incentivise the research and development required for new medicines and treatments to become available for HTA (noting that there are substantial risks in the development of innovative treatments and health technologies). Further consultation is recommended to ensure that there is a balanced approach to horizon scanning, and stakeholder expectations in terms of viable healthcare solutions successfully navigating HTA are managed accordingly." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was support from these stakeholder groups with a focus on the significance of jurisdictions, patient and clinician involvement, not narrowing the focus of the horizon scanning activities and forming international collaborations.

"I thought that it was a good idea to form international collaborations with trusted groups with similar value systems." (The University of Notre Dame Australia)

"Australia could become a world leader in the development of consistent processes to measure health equity considerations in HTA for priority populations such as First Nations people in all

phases throughout the HTA decision-making process. A publicly available calculator has been developed for the UK context for socioeconomic quintiles and can assess the likely direction and size of health inequality impacts of interventions under consideration. It allows a quick indication of whether health equity impact might be decision relevant, and whether further analysis is required. Calculators could measure the impact of interventions by Indigenous status, area of remoteness index and socioeconomic position. The development of these economic tools for priority populations in Australia will enable decision-makers to know the full picture of health equity impacts. We will begin to develop Australian versions of the health equity impact calculator in 2024. The calculator could be used at the initial stage of scoping and early assessment of new technologies where initial advice is being collected to guide discussions on the direction and significance of health inequality impacts. This economic tool should be part of a framework that will provide a transparent, rigorous evidence-informed approach to ensure that funding decisions do not increase health inequalities and where possible reduce health inequalities amongst priority populations." (Deakin University)

"Horizon scanning activities should include the full scope of the IVD sector, not just those technologies within the narrow scope of medicines." (Pathology Technology Australia)

"Involving patient and clinician communities in the identification process ensures diverse perspectives are considered, enhancing the relevance and effectiveness of the scanning efforts." (Society of Hospital Pharmacists of Australia)

"In agreement, noting the need for jurisdictional consultation and feedback. Additionally, how does this align with the abovementioned priority listing? 1. Enable consumer- and/or clinician-initiated submissions for repurposed off-patent medicines that are listed on the PBS for other indications. Submissions should be required to be accompanied by sufficient researched scientific evidence of both clinical efficacy and health system economic impact to facilitate an adequate preliminary assessment; the PBAC would decide to request or decline a review to be undertaken a qualified team for a full submission for consideration of listing change. 2. Regularly invite disease specialists to submit treatments identified in their practice/research that has strong or growing evidence of success in off-patent repurposed use. When there are multiple clinicians indicating the same treatment, it warrants investigation by the PBAC. Number 1 cannot be tied to number 2." (Consumer and independent researcher)

Table 85. Horizon Scanning to help operational and capacity planning for HTA and health systems – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	31%	63%	6%	16
Pharmaceutical / Medical technology company	0%	0%	29%	57%	14%	0%	14
University or research sector	0%	0%	50%	25%	25%	0%	4
Industry association / Peak body	0%	0%	14%	57%	29%	0%	7
Clinician (or representative organisation)	0%	0%	0%	100%	0%	0%	1
Consulting	0%	0%	0%	100%	0%	0%	1
State / Territory government	0%	0%	100%	0%	0%	0%	1
Other	0%	0%	50%	25%	25%	0%	4

Patients, Consumers and Representative Groups

There was widespread support from these stakeholder groups for this option.

"We support the establishment of horizon scanning programs to ensure Australia's HTA system is aware of and responsive to emerging technologies or patterns of use." (Asthma Australia)

"Gene therapy in haemophilia is one example where work to prepare the ground to help operational and capacity planning is taking place in Australia, learning from the process in other countries :

- *Australia has participated in the international clinical trials*
- *The World Federation of Haemophilia (WFH) has developed a Gene Therapy Registry to aggregate clinical results internationally*
- *WFH has also developed a Shared Decision-Making Tool for patients and clinicians considering new haemophilia therapies and this will be tested in Australia*
- *The Australian Haemophilia Centre Directors' Organisation (AHCDO) has developed a model of care to provide equitable best practice access to gene therapy nationally*
- *PROBE international is developing and validating patient reported outcome measures specific to gene therapy in haemophilia, with participation from Australian patients*
- *HFA and Australian clinicians have been looking closely at patient outcomes and experiences reported in Australia and other countries*

- *Australian stakeholders are monitoring the progress of gene therapy for haemophilia through HTA in other countries with similar health economies, consulting with international colleagues on their experience and are considering what may be required for HTA in Australia."* (Haemophilia Foundation Australia)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology Companies stated support for this option with an emphasis that more information is needed and some further suggestions could be considered.

"Horizon scanning can certainly help with capacity planning for HTA systems and health system planning, but the requirements of these two systems are very different, and so developing the same objectives for the two systems is not appropriate. For example, identification of therapeutic advances 18-24 months prior to entry into the regulatory or reimbursement system may be appropriate for submission processing capacity planning and for budget planning, but not for the development or changes to infrastructure to enable implementation of the therapeutic advances." (Eli Lilly Australia)

"Further clarity and detail is required to better understand the impact of horizon scanning. Roche strongly supports:

- *The Commonwealth taking a lead role which extends to securing Commonwealth funding for implementation of horizon scanning on behalf of the States and Territories which can be further detailed in the next NHRA,*

- *A collaborative approach with industry to accelerate establishment of horizon scanning, as opposed to industry providing advanced notice as proposed in the options*

- *A flexible time horizon not fixed at 18-24 months but calibrated according to the level of disruption expected. For example, longer lead times may be required for significant changes to workforce capability and capacity or investment in new complex infrastructure.*

- *Further rationale for cost recovery being proposed for international collaboration when as noted in the options paper, "horizon scanning in the healthcare context can be taken to broadly describe a process that is intended to help different stakeholders be aware of the implications of technologies that will affect healthcare policy or delivery in some way, and (where necessary) provide an evidence base to support the case for changes to the health system in some form." As these activities would have a benefit to multiple stakeholders (not just sponsors) it is not reasonable for the full cost of horizon scanning to be recovered from industry."* (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was broad support from these stakeholder groups.

"Again, I thought that including a capacity to measure and evaluate this was good." (The University of Notre Dame Australia)

"By identifying major therapeutic advances and trends, this option facilitates proactive planning for regulatory and reimbursement systems and enables anticipation of potential disruptions in treatment paradigms. Collaboration with industry ensures a comprehensive approach to horizon scanning, exploring alternative mechanisms if necessary to enhance effectiveness. This proactive approach supports efficient resource allocation and system preparedness, ultimately improving decision-making and healthcare delivery in the HTA process." - Society of Hospital Pharmacists of Australia

5.3. Consideration of environmental impacts in the HTA

Overall, stakeholders welcomed potential greater inclusion of environmental impacts being considered in the HTA process. A number of patient representative groups, peak bodies, clinicians and researchers highlight the impact that the healthcare system has on climate change and the environment. It is also mentioned that climate change and increased pollution have a significant impact on the health and wellbeing of patients and consumers (with asthma sufferers put forward as a key example).

Many stakeholders focussed on the need to consider environmental impacts through all stages of HTA processes to futureproof the system. Stakeholders discussed environmental impacts being reported throughout assessments and particularly as part of the cost-effectiveness considerations.

Table 86. 5.3. Consideration of environmental impacts in the HTA: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	67%	0%	0%	33%	6
Pharmaceutical / Medical technology company	0%	29%	18%	47%	6%	17
University or research sector	0%	73%	27%	0%	0%	11
Industry association / Peak body	0%	57%	43%	0%	0%	7
Clinician (or representative organisation)	25%	0%	75%	0%	0%	4
Consulting	0%	0%	0%	0%	100%	1
State / Territory government						0
Other	25%	50%	25%	0%	0%	4

Patients, Consumers and Representative Groups

There was some support from these stakeholders for the inclusion of environment impacts in the HTA. This was particularly evident from patient representative groups whose cohorts health are significantly impacted by climate change.

"Climate change mitigation is an advocacy priority of Asthma Australia and measures that reduce Australia's emissions are urgently needed." (Asthma Australia)

"It is strategic and proactive for environmental issues to be considered" (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

There was broad support from these stakeholders for the inclusion of environment impacts in the HTA and the urgent need to address the health risks associated with climate change.

"AZ supports efforts to include EIA in HTA evaluation. AZ is aligned with Australia's first National Health and Climate Strategy that there is urgent need to address the health risks associated with climate change. A key action in the Strategy involves the inclusion of emissions considerations in HTA evaluations, starting with embodied greenhouse gas emissions. The Options paper outlines activities such as examining the potential for use of these data in approval and reimbursement decisions, development of guidance documents and examples and alignment with international best practice in comparable jurisdictions. AZ believes these options are positive, however, the inclusion of EIA information should not slow time to access, or overly burden with HTA process with data requirements." (AstraZeneca)

"While the principle of addressing environmental impacts of health technologies could merit further consideration, this must be developed carefully to avoid unintended consequences that could hinder or cause delays in access. Until methods for reporting environmental impact are

more established, inclusion of these data in cost effectiveness or reimbursement decisions would be premature. Determination of evaluation criteria or proposals should include careful consultation with industry as a key stakeholder to ensure reporting requirements are fit for purpose, meaningful, achievable and add value." (Pfizer)

"The framework for environmental sustainability considerations should be workshopped together with the pharmaceutical sector to ensure any approach is aligned with global objectives. Further, the inclusion of environmental concerns should be part of the qualitative assessment as an incentive, rather than as a requirement or any other element that can penalise companies (given that much of the environmental sustainability concerns would be outside the scope of local stakeholders)." (UCB Australia)

"Roche is proud to be recognised as one of the most sustainable healthcare companies in the Dow Jones Sustainability Indices since 2009 and is mindful of its environmental footprint. If introduced, the potential weighting of environmental impacts in the decision making process would need to be clarified. There is a risk of adding further complexity to the evaluation process with the inclusion of environmental impact components, particularly whereby corporations are already bound by other existing legislative requirements to report and deliver on environmental impact targets. It is unclear how including the environmental impacts in HTA will improve timely access to new health technologies." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was overwhelming support for the consideration of environmental impacts amongst these stakeholder groups. Stakeholders stated an emphasis on the urgency of this issue drawing attention to the stated very large carbon and waste footprint of the Australian healthcare system.

"The proposed options do well to address their related issues. If implemented, the options would also be world leading. For environmental impacts to be included as not only part of the HTA process, but also, for the emissions data of health technology products to be publicly available would directly address the challenge and opportunity to safely decarbonise Australia's health system. Such information would enable patients and clinicians to make low emissions decisions on medicines and medical technologies when other factors, such as clinical outcomes and cost effectiveness, are equal. Ultimately, this could influence industry competition towards sustainable, low carbon technologies. Also, reducing the health system's carbon emissions is a key objective of Australia's National Health and Climate Strategy. Implementing these options directly corresponds to several of the Strategy's actions, in particular, action 5.1 "Considering the role for emissions footprinting of health technology products The Australian Government will, in consultation with industry and other relevant stakeholders, review options for including public reporting and consideration of environmental impacts, starting with greenhouse gas emissions, of health technologies, in collaboration and alignment with international best practice in comparable jurisdictions." (Public Health Association Australia)

"AHHA has long advocated for, and strongly supports, the embedding of environmental considerations into the prosses of HTA in Australia. Prioritising new technologies with a low carbon footprint is a simple strategy to reduce the health and aged care's impact on climate change and will ensure alignment with the National Health and Climate Strategy in particular Objectives 2 (Health system decarbonisation) and 3 (International collaboration). Additionally, organisations, services and health professionals across Australia are actively engaging in efforts

to reduce the environmental impacts of the care they provide, yet report difficulties due to the complexity of obtaining comprehensive information to inform procurement and clinical decision around scope 2 and 3 emissions on top of already significant workloads. Activity to assess the environmental impact of new health technologies and products at the HTA level and transparency of reporting of this information is a critical step to better supporting decarbonisation and investment decision making at all levels of the health system." (Australian Healthcare and Hospitals Association)

"The options suggested here would represent an important step forward in this increasingly important and urgent space. However, it is critical to recognise that HTA must be treated as just one component of an integrated, overall climate and health strategy. HTA has a very important role to play but must be fully integrated with other aspects of the drive towards low carbon, climate resilient health systems. Many other regulatory tools (e.g. overall product standards) might actually be more direct and cost-effective tools for reducing the carbon footprint of products than would making HTA the only mechanism. I understand the importance of addressing climate and greenhouse gas emissions but would urge the Review not to discount other key environmental impacts, especially biodiversity loss (particularly when key active ingredients may be sourced from at risk species or ecosystems) and the impact of other forms of pollution." (Menzies Institute for Medical Research, University of Tasmania; Member, South Australian Health Performance Council)

"Healthcare in Australia has a very large carbon and waste footprint. I teach a course on the environmental sustainability of health care and Healthcare systems and the healthcare workers who attend this are deeply concerned about the carbon and waste footprints of their practice. While as individuals they can support and help drive change locally - the scale of the issue requires system level solutions. An individual pharmacist in a country hospital has only so much influence on the carbon footprint of inhalers. We need change at pace and scale if we are to meet national targets for containing carbon emissions. This is an essential development described in this section." (The University of Notre Dame Australia)

"A research agenda is required to ensure process-based life cycle assessment is undertaken for commonly used products, and that a health services research agenda is supported to evaluate consequences of these reforms." (Health Services Research Association of Australia and New Zealand)

"It is clear that action on climate change is hugely beneficial for social, environmental, cultural and economic outcomes. However, these benefits can only be achieved with urgent and decisive action, coupled with the government policy and funding to execute it. The recent launch of Australia's first National Health and Climate Strategy (NHCS) is a significant step forward in addressing the health impacts of climate change and the environmental burden of the health sector. While the NHCS needs funding to undertake its commitments and actions, it also requires a rethink of our health systems policies, systems and procedures. It will take a whole of system approach, whereby each branch and agency within the Department of Health and Aged Care (DOHAC) considers the environmental impacts of its work and seeks to redress the omission of environmental impacts on human health outcomes. As such, CAHA supports the inclusion of 'Consideration of environmental impacts in the HTA' in Consultation Paper 2. This inclusion supports the implementation of Objectives 2 (Health system decarbonisation) and 3 (International collaboration) within the NHCS and are a positive step towards actioning both objectives." (Climate and Health Alliance)

Table 87. Environmental impact reporting – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	17%	17%	17%	50%	6
Pharmaceutical / Medical technology company	6%	6%	38%	19%	6%	25%	16
University or research sector	0%	0%	22%	11%	67%	0%	9
Industry association / Peak body	0%	14%	29%	43%	14%	0%	7
Clinician (or representative organisation)	0%	0%	25%	0%	75%	0%	4
Consulting	0%	0%	0%	0%	0%	100%	1
State / Territory government							0
Other	0%	0%	60%	0%	40%	0%	5

Patients, Consumers and Representative Groups

There was some support from these groups for the option to report environmental impacts, however some responded that they did not know what the impact would be on their organisation, and they highlighted the need for more specific information before they could support the option. Stakeholders called for more consultation on this option.

"Given the particularly harmful impact that the effects of climate change have on the health of people with asthma and the significant contribution of health technologies to climate change, appropriately resourced, planned and co-designed environmental impact reporting could be very positive on consumers and Asthma Australia." (Asthma Australia)

"In principle, CHF welcomes and supports environmental impact reporting. However, the options in this section are too vague and will require more specific consultation prior to design and implementation." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

Pharmaceutical / Medical Technology companies were supportive of the need and premise of environmental reporting but were not sure how this contributed to accelerated patient access. One company proposed that environmental planning should be managed under TGA requirements and streamlined through one government department. Companies recommended careful and considerable consultation with industry to determine requirements and the appropriate timing to establish them.

"Boehringer Ingelheim supports the underlying premise of the need to embed sustainability across all industries. However, further detail is required to understand the proposed environmental impact reporting obligations. Furthermore, any proposed changes should not make the submission process more onerous and detract from the fundamental issue of faster patient access." (Boehringer Ingelheim)

"Environmental impact reporting should be managed under TGA requirements, including manufacturing standards, including packaging. All of the points mentioned are valid, however if we can streamline through one government department that would be ideal, because the same data would be reported and would only add to duplication." (Antengene Australia)

"This potentially more work for companies that are largely neutral, and difficult to see how this contributes to more timely access." (Amgen)

"While environment impact reporting is important, this itself does not contribute to accelerated patient access and therefore is not considered relevant to the HTA Review or a priority for implementation." (Eli Lilly Australia)

"*Bayer is committed to environmental sustainability; however, this option needs further consideration and development to ensure measures are aligned globally. This option should be a qualitative consideration with a focus on incentivizing sponsors to maintain responsible environmental policies rather than penalizing them. Inclusion of environmental impact into cost effectiveness analysis may increase uncertainty in the value of medicine, therefore this should remain an option element within economic analysis."* (Bayer Pharmaceuticals ANZ)

"Whilst there are some potential positive elements to this option such as - greater awareness of environmental considerations, it is unclear how these will be measured or if greater resourcing is required to implement environmental impacts as outlined in the options. We note that, whilst reporting carbon emissions related to a technology related to an asthma inhaler may be relatively more straightforward, factoring in carbon emissions and quantifying the environmental impact externalities could be a substantial resource requirement. Furthermore, the added requirement of reporting and monitoring may be administratively burdensome. Guidelines would need to be standardised into recording and reporting mechanisms to ensure reporting is meaningful, including an agreed calculation method. An example of an area where further clarity is required is how environmental impact elements might be weighted in the decision-making process, and the effect the collection of this data might have on timely access to new health technologies. Roche recommends further consultation so that all stakeholders can better understand, have greater clarity and co-design potential alternative approaches where necessary, noting the broader whole of Government requirements for environmental impact reporting." (Roche Products)

"Menarini supports embedding sustainability across all industries. This option would need to be developed in close consultation with the pharmaceutical sector to ensure measures are aligned globally, given the global nature of the industry. It should be a qualitative rather than quantitative consideration, with a focus on incentivising sponsors. Additionally, this option should not slow patient access." (A.Menarini Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

This option was seen as essential and critical to these stakeholder groups. They have provided significant comment in support of environmental reporting.

"Reporting will provide critical additional data and I would strongly welcome it. But it must be firmly based on international standards and best practice, and well-integrated with other environmental reporting requirements on firms. Avoid getting caught up in semantic arguments

about Scope 1/2/3 and onshore versus offshore emissions etc. – reporting must capture full carbon (or other environmental) footprint of products as manufactured and used. Effective reporting must be mandatory and not voluntary, and to standards acceptable to Government, not just using industry ESG metrics." (Menzies Institute for Medical Research, University of Tasmania; Member, South Australian Health Performance Council)

"Agree. Prioritise the use of process-based life cycle assessment (LCA), which is precise, robust and evidence based. Ensure that scope 2 emissions are accurately captured and included in reporting" (Doctors for the Environment Australia)

"Reporting greenhouse gas emissions is essential. Both benefits described on page 159 of the review document are very important. The example for the inhalers is excellent. Having international alignment is essential. We all have scare resources and with agreed approaches resources can be combined. The companies producing these products are international companies and we need an international and consistent response to them." (The University of Notre Dame Australia)

"A core goal of the PHAA is to see an effective response to climate change and its impact on health. The National Health and Climate Strategy (NHCS) is an important conduit to mitigating climate change as well as beginning the process of adapting to the change that is already occurring. Reducing health system emissions is a core objective of the NHCS and emissions footprinting of health technology products are specifically outlined as one action to achieve this objective. If implemented, these options play a key part in the larger effort to reduce the health system's emissions. The health system is responsible for 5.3% of Australia's emissions, so reducing emissions in the sector is an important factor in reducing Australia's total emissions. However, PHAA acknowledges that most emissions reductions must be made by the highest emitting sectors including energy, stationary energy, transport and agriculture to reach Australia's Paris Climate Agreement target. Rapid reduction of emissions would mean mitigating the global temperature increase to well below 2°C above pre-industrial levels and limit it to 1.5°C above pre-industrial levels. Achieving this would drastically reduce the worst impacts to health, the environment, food sustainability, infrastructure, and the list continues. Implementing the evidence informed actions of the NHCS to protect the health and wellbeing of Australians is not only a positive outcome for PHAA, but is a positive outcome for all." (Public Health Association Australia)

"There is no doubt that further environmental impact reporting will add regulatory burden to companies. However, this doesn't mean it should happen given the wider social and health issues involved which MedTech are committed to addressing. However, it needs to be reasonable and in line with global standards given that most products are imported and part of a global supply chain. Furthermore, patient outcomes should remain the priority when determining reimbursement." (Medical Technology Association of Australia)

"By investigating the feasibility of reporting embodied greenhouse gas emissions and other environmental impacts, HTA bodies can contribute to broader efforts to mitigate climate change and promote sustainability in healthcare." (Society of Hospital Pharmacists of Australia)

"All companies (commercial and not-for-profit) will need to adopt the ESG accounting standards in coming year(s). planning now for how this will impact the decision making and horizon

scanning is key." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

5.4. Mechanisms for continuous review and improvement;

Table 88. 5.4. Mechanisms for continuous review and improvement: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	7%	79%	0%	14%	0%	14
Pharmaceutical / Medical technology company	0%	27%	53%	13%	7%	15
University or research sector	0%	50%	0%	0%	50%	2
Industry association / Peak body	14%	43%	29%	0%	14%	7
Clinician (or representative organisation)	0%	50%	50%	0%	0%	2
Consulting	0%	0%	100%	0%	0%	1
State / Territory government						0
Other	0%	75%	0%	0%	25%	4

There was strong support amongst stakeholders for the reforms in Option 5.4, particularly in respect to proposed transparency and greater forward planning of consultation and review. Many stakeholders mentioned continuous review and improvement as pivotal to the long-term success of the HTA and to constantly be able to meet the needs of a rapidly evolving and technology driven system. As technologies and treatments change and innovate, the pharmaceutical and research stakeholders emphasised the importance of the system having adequate flexibility to accommodate assessment of these new technologies and explicit KPIs to track the success of any new reforms.

Patients, Consumers and Representative Groups

These stakeholder groups were supportive of these options and welcomed mechanisms for review more frequently. Those who were not sure about this option, highlighted a stated need for the methods to be explicitly clear and timelines to be defined for stakeholders to follow.

"This option should provide a mechanism for HTA processes to evolve with innovation in health technologies." (Rare Voices Australia)

"These methods must be explicitly outlined and clear for all stakeholders to follow." (NeuroEndocrine Cancer Australia)

"The solution proposed appears to be very limited and does not specifically address the complexities of pro-actively engaging consumers and other relevant stakeholder groups to

support continuous improvement. Guidelines that are constantly evolving with the latest information and updates must be easily accessible, easy to read and understand, relevant and timely. Setting realistic timeframes for review including allowing adequate time horizons for stakeholder contributions must be more clearly articulated in terms of continuous review and improvement." (Anonymous submission)

Pharmaceutical / Medical Technology Companies

There was general support for these options, but more detail and explicit KPIs were requested.

"Supportive however there is lack of detail and industry involvement." (Novartis Australia)

"For successful review and improvement, key performance indicators need to be defined." (UCB Australia)

"GSK agrees with Medicines Australia that in the interests of continuous review and improvement, it will be crucial to embed agreed KPIs in the mechanisms. There needs to be a measure for access that is agreed by all stakeholders, so that progress can be meaningfully measure." (GSK)

"AZ believes major HTA system reviews should occur at shorter time intervals as part of a process of rolling reviews. Moreover, an agreed set of KPIs need to be developed with stakeholders to objectively measure how the HTA system is performing. The Options paper notes the Department publishes PBS process statistics each year and has committed to working with Medicines Australia to determine a range of KPIs. KPI development is a priority, otherwise improvement in the HTA system cannot be objectively measured." (AstraZeneca)

"The proposed options would mostly address the issue of a continuous review and update to guidelines to ensure alignment and relevance with the newest health technologies. However, the suggested options require more detail to enable assessment and determine whether they will be fit for purpose and resourced appropriately." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was some support for these options amongst these stakeholder groups, but they did not provide extensive commentary.

Table 89. A program of continuous review and improvement for current HTA policies and methods – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	7%	0%	0%	29%	64%	0%	14
Pharmaceutical / Medical technology company	0%	0%	13%	67%	7%	13%	15
University or research sector	0%	0%	50%	50%	0%	0%	2
Industry association / Peak body	0%	0%	0%	71%	29%	0%	7
Clinician (or representative organisation)	0%	0%	50%	0%	50%	0%	2
Consulting	0%	0%	0%	100%	0%	0%	1
State / Territory government							0
Other	0%	0%	25%	50%	25%	0%	4

Patients, Consumers and Representative Groups

There was overwhelming support for this option from these stakeholder groups, but one group highlighted some concern that is outlined below.

"Regarding mechanisms for continuous review and improvement (5.4)—Painaustralia strongly supports a program of continuous review and improvement for current HTA policies and methods. The effective implementation of the five proposed program components will facilitate earlier patient access to therapeutic innovations in a timely, equitable, safe and affordable way." (Painaustralia)

"CHF will always support plans for the continuous evaluation of HTA processes. Care must be taken to ensure that such evaluation includes qualitative data, and is collected from a suitable variety of sources. Most importantly, the evaluation must not mistake HTA outcomes with HTA outputs. Naturally, capturing short, medium and long term outcomes of HTA outputs will require adequate funding." (Consumers Health Forum of Australia)

"Sustainable from one government to next is important." (NACCHO)

"Timeframes and compliance are a big downfall in all domains. Stakeholders should deliver on time in order to make treatments accessible. Language choices in reporting are also important. I've not spoken elsewhere perhaps about the fall in peer review standard and the inaccurate and fatalistic prognosis information that pervades even the newest technological approaches, by not acknowledging the efforts of clinicians for the last decade or more and worse, not giving patients an accurate foundation/information on which to base ethical decisions about affected children and their prognosis and therefore treatment choices. Critical literacy is so important and a skill for consumer representatives to consolidate - much easier if our population returned to a basic health science foundational curriculum through school. The problem is only going to worsen with

Ai, aggregating from both grey and medical literature, very out of date abilities, incidence, survival rate etc." (Save Our Sons Duchenne Foundation)

Pharmaceutical / Medical Technology Companies

There was broad support for these options, with further caveats for the inclusion of KPIs and ongoing consultation with industry, particularly with Medicines Australia – in line with current practice.

"Boehringer Ingelheim supports the concept of continuous improvement. However, there must be a consultation period with Medicines Australia whenever changes are made to the PBAC guidelines, as this has been the established process thus far." (Boehringer Ingelheim)

"Continuous review and improvement programs should be embedded with agreed key performance indicators that meet the objectives of the Strategic Agreement so that processes can be meaningfully measured. The implementation of co-designed key metrics was highlighted as a facilitator of earlier patient access in the Strategic Agreement (clause 6.1: Continuous process improvement). These metrics include 'Reduce time to PBS listing, including time from TGA registration to PBS listing within the Term of the Agreement'. A baseline measure will be established, and specific metrics will be reported on an ongoing basis. The implementation and publication of performance indicators was also raised through the Parliamentary Inquiry's The New Frontier report. In line with MSD's recommendation from Consultation 1, these metrics should be categorised as:

- *domestic; capturing the following milestone dates:*
    - *ARTG listing to PBS listing,*
    - *TGA submission to PBAC submission,*
    - *PBAC submission date to PBAC recommendation, and*
    - *PBAC recommendation to PBS listing.*
- *international; from the date of the earliest marketing or regulatory authorization amongst OECD countries (or another pre-specified group of countries) until PBS listing date.*

These metrics should be published on a PBS webpage such as the Medicine Status Website (MSW) and updated routinely (at least twice per year)." (MSD Australia)

"A process of continual review and improvement is valuable and should have clear objectives and KPIs which align with the goals of the current HTA review to reduce time to access for Australians, maintain attractiveness as a first launch country and ensure processes keep pace with advancing technologies. The risk without defining these goals and agreeing on KPIs, is that such a process could become a vehicle for regular price policy negotiations which would run counter to the objectives of the HTA review by undermining value and delaying launch in Australia." (Pfizer)

"*Bayer supports activities designed will lead to continuous review and system improvement and it is important that key performance indicators are agreed and embedded. Additionally, a*

measure for medicines access that is agreed by all stakeholders needs to be defined so that progress can be meaningfully measured." (Bayer Pharmaceuticals ANZ)

"Roche supports in principle options which address the outlined need for a continuous approach to reviewing and updating guidelines, methods, policies and processes, so that HTA in Australia can keep pace with the evolution in health technologies. Roche notes that more clarity is required to understand the parameters of the proposed reviews, implementation of potential findings, expectations and contributions from industry and other stakeholders, and resourcing requirements. Roche has previously noted that more guidelines does not necessarily translate to improved or accelerated access, especially in the circumstance that the valuation of technologies is not befitting that of a first-wave country. Additionally, any reporting should outline specific measurements on the policy and method changes implemented as a result of this Review. This will allow both successes and failures to be assessed against the agreed intent in an open and transparent manner with input from all relevant stakeholders." (Roche Products)

"This proposal must include agreed-upon metrics with defined key performance indicators and must align with work being undertaken as part of Appendix 3 of the Strategic Agreement." (A.Menarini Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

A very strong level of support for these options was expressed by these stakeholder groups.

"Overall, this is welcome if applied to HTA for all technologies included MedTech and digital health (unlike this review). Changes need to be monitored for their actual impact." (Medical Technology Association of Australia)

"By systematically evaluating and updating HTA practices, this program can enhance decision-making, promote transparency, and accommodate advancements in technology and methodologies." (Society of Hospital Pharmacists of Australia)

"Concept is supported but how this is operationalised with the reform may need to be assessed again at later day i.e. 12-15m after reform implementation. The forward schedule for review should be stipulated at the time of acceptance of a new HT." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

"I like the idea of continuously reviewing the PBAC guidelines. However, it feels like the industry is forever in the cycle of writing and reviewing PBAC submissions that there isn't the resources to do this effectively. With this in mind, I think it is important that the Guidelines themselves be written and interpreted in a way that doesn't necessarily require continuous review and they are future proofed. As discussed in a previous response, this is why I don't necessarily like the idea of curated methodologies." (THEMA Consulting)

5.5. Capacity and capability of the HTA system

Across stakeholders, and a number of responses to options, the capacity, capability and resourcing of the HTA system was mentioned. There were concerns raised in regard to the capacity of the HTA committees if streamlining were to be agreed and implemented and there have been stated concerns about resourcing and capacity for horizon scanning to be introduced effectively and systematically. There was stated general support for a review and overhaul of resourcing of the HTA system. Any additional concerns that were not covered under the appropriate sections and feedback about the proposed internship program are mentioned below.

Table 90. 5.5. Capacity and capability of the HTA system: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	56%	22%	11%	11%	9
Pharmaceutical / Medical technology company	0%	38%	38%	15%	8%	13
University or research sector	0%	40%	60%	0%	0%	5
Industry association / Peak body	0%	40%	60%	0%	0%	5
Clinician (or representative organisation)	0%	0%	100%	0%	0%	2
Consulting	0%	33%	67%	0%	0%	3
State / Territory government						0
Other	0%	50%	50%	0%	0%	2

Patients, Consumers and Representative Groups

There was general support for an increased HTA capacity and workforce amongst these stakeholder groups, but one group observed that the issues outlined in the paper do not seem to adequately address in these options.

"The scale of the capacity and capability issues outlined by the Options Paper do not seem to have been adequately addressed by the options presented. While we support the measure, the development of a sponsored internship program should not be seen as a cheap way to resource the HTA and interns should be appropriately compensated for their work." (Asthma Australia)

"The speed of innovation and increasing complexity of health technologies requires increased HTA capacity and workforce." (Rare Voices Australia)

Pharmaceutical / Medical Technology Companies

There was support from companies for the strengthening of the capacity and workforce of the HTA. More information was requested on the implementation of this option including timelines and how the resourcing and training were going to be organised.

"The Options paper proposes work-sharing locally and internationally as strategies to improve HTA evaluation efficiency. These options have value, although a key constraint is current limited HTA capacity and workforce in Australia. AZ believe there is need to strengthen this workforce with specialist expertise in HTA methodology and policy. The option of developing a sponsored internship program with universities and industry using secondment positions is a positive step. Funding case manager positions is suggested in the Options Paper. International workshare could help improve HTA efficiencies, although it is not clear how the option will be implemented." (AstraZeneca)

"No details are provided on how the resourcing and training are going to be organised and timelines are also unclear." (UCB Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Stakeholders stated support for these options, with an acknowledgement of the stated need to expand the capacity of the HTA, they also highlighted stated skill limitations in the wider workforce as a risk to the implementation of this option. Stakeholders suggested that Commonwealth could support places on public health coursework and that outsourcing could also assist with some of the challenges in the implementation of these options.

"Strongly agree that we need greater capacity in health economics. we also need greater capacity to be able to do environmental assessments of all technologies and indeed all models of care. We don't have the skills in the current workforce to do this. The capacity to respond to these recommendations in this important review document will be limited by the capacity of the workforce. The internship program is potentially one small part of a response - as long as you appreciate that this measure alone will be medium to long term strategy. You might need to actively recruit people with the skills in the first instance and ramp up training across the board in segments of the health workforce." (The University of Notre Dame Australia)

"Suggest also Commonwealth Supported Places are offered for Public Health coursework degrees and Health economics coursework degrees." (Adelaide Health Technology Assessment)

"Contract out research projects to epidemiologists, health economists, and biostatisticians in academic institutions, and through research peak bodies such as HSRAANZ, ACTA." (Health Services Research Association of Australia and New Zealand)

Table 91. Improve HTA capacity and workforce in Australia – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	10%	0%	0%	10%	70%	10%	10
Pharmaceutical / Medical technology company	0%	0%	23%	62%	8%	8%	13
University or research sector	0%	0%	25%	25%	50%	0%	4
Industry association / Peak body	0%	0%	0%	83%	17%	0%	6
Clinician (or representative organisation)	0%	0%	50%	50%	0%	0%	2
Consulting	0%	0%	67%	33%	0%	0%	3
State / Territory government							0
Other	0%	0%	33%	33%	33%	0%	3

Patients, Consumers and Representative Groups

This option was widely supported by these groups.

"With the breadth of scope of the current HTA review, and the sizeable amount of resources and funds it will command, it is paramount for the HTA review to make plans to train and expand the HTA workforce." (Consumers Health Forum of Australia)

"An HTA that has optimal capacity and workforce will be of benefit to consumers by helping to ensure that they have access to health technologies in the shortest timeframe possible, and by helping to ensure that risk, safety, equity and other values such as the environment have all been appropriately considered and accommodated in this process. However, this will require appropriate resourcing." (Asthma Australia)

"CCA support sponsored internships and we suggest that these are targeted to be inclusive of a range of cultural and minority groups to ensure promote diversity across the HTA system." (Crohn's & Colitis Australia)

"Building consumer capacity is essential for consumers to be able to provide informed and meaningful into HTA processes. The HTA Consumer Evidence and Engagement Unit has existing strengths in this areas and should be resourced and authorised to expand this work." (Rare Voices Australia)

"Essential that this improved capacity reflects the National Medicines Policy, the increasing health needs of Australians, and that consumer consultation is fully funded and resourced nationally." (NeuroEndocrine Cancer Australia)

Pharmaceutical / Medical Technology Companies

There was stated support for the increased capacity and workforce of the HTA, they support investment in training courses and offer to share insights from the industry's capability building endeavours. Stakeholders discussed examining what the overall skilled workforce across the health system can bring to the table to support capacity and capability building.

"Development of additional local HTA expertise and workforce capacity could support more efficient assessment and decision making and therefore accelerated patient access. Investment in training courses, both tertiary and post-qualification, from a range of institutions that teach skills useful in the sector would a means of further supporting any internship model." (Pfizer)

"As capacity will be fundamental to the successful implementation of many of these Options, (AbbVie supports the proposal to improve HTA capacity and workforce in Australia and considers that industry is a key partner in achieving this goal. Industry can share insights and successes from prior programs and could also be involved in designing and executing future scholarships, internships, and training programs. The Options Paper proposes to improve HTA capacity and workforce in Australia through the support of students to undergo formal training and internships. (AbbVie notes that several members of the Medicines Industry currently support and fund similar programs, contributing to the development of HTA knowledge and competencies in Australia. For example, many Sponsors operate internship programs or support local health economic research through scholarships such as the Macquarie University Australian Pharmaceutical Scholarship (MUAPS) Program." (AbbVie)

"Roche supports in principle options to improve HTA capacity and workforce in Australia. Consideration should be given to ensuring this is across all sectors from government, evaluation and industry sectors as each brings their own unique skills, expertise and perspectives to furthering healthcare in Australia." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These groups stated broad support for this option and highlighted the stated significant challenges facing the HTA to build that skilled workforce.

"Without a skilled workforce it will be difficult to achieve the necessary outcomes. The need to assess these technologies from an environmental perspective is a rapidly emerging need and one that Australia must respond to. The lack of an adequate health economics workforce has been a long-standing issue. The other skill that is not mentioned here is a capacity to take a systems approach to these issues. Systems thinking does not currently feature in health professional development." (The University of Notre Dame Australia)

"This is a significant challenge. Where does the workforce come from i.e. professional development pathways and incentives to retain talent in Australia are an absolute priority. Support the proposed internship as a pathway to increase the numbers of appropriately qualified and experienced candidates." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand)

5.6. Strengthen international partnerships and work-sharing

In some instances, stakeholder groups agreed that there were benefits from international partnerships and work sharing, but there were particular topics where groups highlighted some concerns. These stakeholders did not endorse or see the benefit of international purchasing or buying groups and there was a call generally across stakeholder groups for much more detail and consultation on these options.

Table 92. 5.6. Strengthen international partnerships and work-sharing into account: How well reforms address issues by stakeholder type

	Completely address the issue(s)	Mostly address the issue(s)	Address some but not most of the issue(s)	Address little or none of the issue(s)	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	73%	18%	0%	9%	11
Pharmaceutical / Medical technology company	0%	0%	53%	47%	0%	17
University or research sector	25%	25%	25%	25%	0%	4
Industry association / Peak body	0%	43%	43%	14%	0%	7
Clinician (or representative organisation)	0%	33%	67%	0%	0%	3
Consulting	0%	100%	0%	0%	0%	1
State / Territory government	0%	50%	50%	0%	0%	2
Other	0%	67%	0%	33%	0%	3

Patients, Consumers and Representative Groups

There was a high level of support for strengthening international partnerships from these stakeholder groups, many believed this was critical to decreasing the time to access treatments for patients. There was a comment from one of these groups that they could only "mostly" support these options due to the overly technical language describing these in the options paper.

"This is critical to speed up approvals. MND patients do not have time to wait for duplicate approval processes to be undertaken. Jurisdictions should align processes and share data as much as possible." (MND Australia)

"The Australian diabetes community expresses frustration that technologies available in other jurisdictions lags in Australia. While this is often a consequence of commercial decisions by industry, any effort that harmonises approvals with international best practice would be welcomed." (The Australian Diabetes Alliance)

"We support this recommendation for the purposes of improving time to listing and HTA capacity and in particular joint expression of interest pathways for specific rare disease treatments." (Rare Voices Australia)

"Some of the options presented are very technical and hence we deemed it only possible to suggest that they might 'mostly address the issue/s' given that they seem to be sound options and based on the Review's investigative research over past months." (Asthma Australia)

Pharmaceutical / Medical Technology Companies

Many of these companies expressed a belief that these options only somewhat addressed the issues, if at all. There was support for international collaboration, but one particular stated area of concern for industry was the prospect of Australia joining buying groups in international markets. They stated a strong belief that this could actually disadvantage Australian patients in terms of timely access to affordable technologies.

"Alexion opposes this in principle. If Australia were to join a buying group with other markets, it is expected that manufacturers would need to waive rights to confidential pricing among the payers within the buying group to generate a common price. This would have detrimental international reference pricing implications that would be unviable for manufacturers. It would ultimately result in new health technologies simply not coming to Australia." (Alexion)

"This should not appear in the Reference Committee's final report. It is not likely to be feasible for sponsor companies and it is unclear how this will speed up access for Australian patients." (Bayer Pharmaceuticals ANZ)

"BMSA, while not opposed to some of the benefits that might arise from greater international collaboration, is opposed to the option that recommends investigating "opportunities for collaboration with international jurisdictions to increase market share and purchasing power for innovative health technologies which address areas of HUCN". Australia already pays some of the lowest prices in the developed world for innovative medicines. It is hard to see how this initiative would improve matters in terms of appropriate valuation of innovation and speed of access by patients." (Bristol Myers Squibb Australia)

"While we support international collaboration if it leads to enhancement of HTA processes and faster access for patients, this collaboration must not oversee the specific context and nuances of each setting. We do not support any international collaboration that would threaten the confidentiality of prices or clinical data. The overall impact of these options is unknown until further information on implementation is provided." (UCB Australia)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These stakeholder groups were supportive and could reportedly see the benefits of international collaboration, but raised some questions that needed to be addressed as to whether international partnerships would actually advance Australia, as we could actually see an increase the cost of treatments aligning with international partnerships.

"As demonstrated by the COVID-19 pandemic, when we collaborate internationally, many positive outcomes can occur. We need to take away barriers, professionally or legally, in order to reap the benefits of information sharing." (Australasian College for Emergency Medicine)

"International partnerships with trusted countries and organisations that share our values provide an opportunity to combine scarce resources. There are also risks if the countries or groups are

too closely aligned with the private sector. So, some process of screening and regular review of international partnerships is required." (The University of Notre Dame Australia)

"It is unclear whether international work sharing would really be an advance. One test is whether it would reduce cost for the sponsor based on cost recovery. If it doesn't it probably means nothing has been gained." (Medical Technology Association of Australia)

Table 93. Harmonisation of HTA evaluations – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	50%	33%	17%	12
Pharmaceutical / Medical technology company	13%	25%	44%	6%	0%	13%	16
University or research sector	0%	0%	40%	40%	20%	0%	5
Industry association / Peak body	0%	0%	13%	75%	0%	13%	8
Clinician (or representative organisation)	0%	0%	33%	67%	0%	0%	3
Consulting	0%	0%	100%	0%	0%	0%	1
State / Territory government	0%	0%	100%	0%	0%	0%	1
Other	0%	0%	33%	67%	0%	0%	3

Patients, Consumers and Representative Groups

There was support for international harmonisation by these groups, they highlighted the potential for preventing costly duplication that this option aims to provide, so long as international processes were as equally vigorous as Australia's. Additionally, one group stated this should be included as part of horizon scanning activities.

"Noting the interconnectedness of many of the recommendations in the Options Paper, BCNA encourages international harmonization to be part of horizon scanning activities to ensure consistency and equity with comparable jurisdictions overseas, as well as the potential to avoid overall duplication of work." (Breast Cancer Network Australia)

"Transparent pathways and information sharing is essential for all stakeholders." (NeuroEndocrine Cancer Australia)

"CHF is not opposed to efforts at harmonising Australian HTA processes with international processes, assuming such processes equally prioritise safety, quality, and efficacy. If done well, harmonisation can prevent costly work duplication and deliver better value-for-money to consumers." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

These companies stated there are potential benefits from international work-sharing, including the ability to increase the capacity and capability of the HTA workforce, improve international consistency and time to listing, but they cautioned that it could lead to more complex and restrictive requirements if not conducted without adequate local consultation with stakeholders.

"Roche supports the harmonisation of HTA across jurisdictions on the basis of improving international consistency, time to listing and HTA capacity. Priorities for the HTA collaborations must be to establish and ensure a streamlined, well-integrated process that improves patient access to innovation across all countries (timely and equitable access), uses a state-of-art assessment approach, and engages with industry, patients, clinicians, academia and other experts throughout the process. Processes need to be appropriately resourced to ensure a clear, workable and predictable framework, delivering consistent high-quality outputs. Roche supports the harmonisation of HTA methods. International alignment on technical matters may result in Australia better understanding the methodological approaches considered best-practice by HTA agencies, such as NICE, leading to wider adoption in Australia. Consequently, this may enable earlier submissions in Australia by reducing the need to respecify base case parameters or develop Australian specific cost-effectiveness models beyond simply adapting specific local costs. To achieve this however, the PBAC and its sub-committees must be willing to soften long held positions where they differ from those; Roche noted this in Consultation 1. The alternative would result in even further delays to submissions and evaluations and would need to be conducted in sequence rather than in parallel." (Roche Products)

"In principle, Menarini is supportive of harmonisation of HTA evaluations but requests that this option does not disadvantage companies who either:

- *are not the licensor for the product in one or more of the jurisdictions which are part of this collaboration, as it may be in breach of competition laws for these companies to collaborate on the applications, or*

- *do not have a legal entity in that jurisdiction and therefore would not be submitting an HTA application in that jurisdiction"* (A.Menarini Australia)

"Harmonisation of methods could be beneficial in some circumstances, but should always be considered against the goals of the HTA review. This proposal has the risk of creating more conservative, restrictive or complex requirements if developed without adequate consultation of local stakeholders including industry." (Pfizer)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

These groups were supportive of this option, they highlighted stated potential benefits such as streamlined processes, timeliness and fostering collaboration. But they also identified some stated risks around resourcing, information sharing of pricing or budget-related information and cultural and values differences between Australia and other countries.

"By aligning evaluation methodologies and facilitating early dialogue with stakeholders, including industry sponsors, clinicians, patients, and regulatory entities, jurisdictions can streamline processes, reduce duplication, and promote consistency in decision-making. This approach not

only enhances efficiency but also fosters collaboration, knowledge exchange, and mutual learning among participating jurisdictions, ultimately leading to more robust and evidence-based HTA outcomes." (Society of Hospital Pharmacists of Australia)

"Proposal appears to address timeliness issues." (Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand

"Interagency working requires resourcing to be successful. These measures must be adequately resourced to obtain full benefit." (The University of Notre Dame Australia)

"While I can see the value in more harmonisation, I am acutely aware that HTA processes have been set up to reflect the health systems, values and cultures in different countries and that these differ between countries (as seen in Paper 1). Harmonisation in methods and processes may not result in harmonisation of funding decisions (as was demonstrated in Europe). Also some methods and processes have values underpinning them that might be contrary to values in another jurisdiction. I am therefore neutral on the subject - I think it will depend on how it is rolled out." (Adelaide Health Technology Assessment)

"It is unclear whether this would take away the flexibility for companies in timing of submissions and whether it would actually reduce resource use. It cannot apply to economic evaluations." (Medical Technology Association of Australia)

Table 94. Work sharing for individual submissions – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	50%	40%	10%	10
Pharmaceutical / Medical technology company	18%	18%	41%	12%	0%	12%	17
University or research sector	0%	0%	40%	40%	20%	0%	5
Industry association / Peak body	0%	0%	17%	67%	0%	17%	6
Clinician (or representative organisation)	0%	0%	33%	67%	0%	0%	3
Consulting	0%	0%	100%	0%	0%	0%	1
State / Territory government	0%	50%	50%	0%	0%	0%	2
Other	0%	0%	50%	50%	0%	0%	2

Patients, Consumers and Representative Groups

Whilst these groups stated strong support for this option, some requested more detail be provided here.

"Essential for complex technologies that have cross jurisdictional funding." (Rare Voices Australia

"More detail is required here." (Anonymous submission)

Pharmaceutical / Medical Technology Companies

There was stated support from these companies for this option, while including a recommendation to consult with Medicines Australia about areas of submissions that should not be subject to collaboration, such as product-specific costs and confidential business information.

"Adequate consultation with Medicines Australia is required to determine which aspects of the submission should be subject to collaboration with other HTA bodies. It is Boehringer Ingelheim's view, that Section 3 and 4 of the PBAC submission are not appropriate to share." (Boehringer Ingelheim)

"Roche supports international collaboration on clinical components of HTA evaluations and would welcome the opportunity to participate in a proposed clinical evaluation pilot. Each of the four pathways proposed have merit and could be appropriate for specific circumstances." (Roche Products)

"Bayer is supportive of international work-sharing for individual submission if it leads to improvements in HTA processes and faster access for patients. Although there are risks within this option that would require further consideration before implementation:

- *Clarity and agreement around the scope and participating markets*
- *Product-specific costs and pricing discussions should be avoided*
- *Confidential business information (bespoke clinical analyses, cost effectiveness analyses, pricing information) would continue to be protected*
- *Consideration of local needs and processes before adoption of international policies."* (Bayer Pharmaceuticals ANZ)

"It's not clear how evaluations from other countries would be accepted in Australia and whether policy differences would mean assessments from other countries don't respond to all the relevant issues. Similarly, there is a risk that the proposal causes additional delay. Without close coordination between countries on priorities, resourcing and processes there is a risk that misalignment of priorities will mean an evaluation that is required in one jurisdiction is delayed by a failure to prioritise it in another. There is a risk that relying on overseas evaluations and commentaries leaves Australian patients and other stakeholders out of the conversation and risks an evaluation being received that has not been informed by patient preferences and needs that aren't aligned with those of Australian patients. Confidentiality obligations must be met to ensure all information that is confidential to one country or shared on a confidential basis with an individual HTA organisation is treated appropriately at all times." (Pfizer)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

Many of these groups supported this option, and believed this collaboration could leverage expertise and build on capability – but with a need for caveats regarding price sharing and budget information.

"As noted, I think this is the only realistic way to leverage the currently embryonic global capabilities in healthcare LCA for environmental assessment." (Menzies Institute for Medical Research, University of Tasmania; Member, South Australian Health Performance Council)

"The literature and experience of workforce and researcher capacity building strongly supports the value of international partnerships. These are beneficial to the organization and to the individual. To the organisation because they strengthen capacity within the unit, widen the potential pool for recruitment and enrich research processes. For the individual they enrich their networks, potentially expand their understanding and thinking and broaden long term employment opportunities." (The University of Notre Dame Australia)

"By piloting initiatives such as the "Work Sharing Initiative" pathway and the "Comparable Overseas Agency" pathway, jurisdictions can leverage each other's expertise and resources to streamline evaluation processes and enhance efficiency in decision-making." (Society of Hospital Pharmacists of Australia)

"While I can see the value in work sharing from an evaluation perspective, I am also aware that HTA processes have been set up to reflect the health systems, values and cultures in different countries and that these differ between countries. Any work sharing will depend on like-minded approaches to assessment and would therefore likely be a slow process - at least early on - and might not result in any efficiencies of process. It will depend on how it is implemented. Work-sharing in terms of concurrent lodgement of submissions to multiple agencies and then work- split among agencies, could result in some efficiencies and early access to medicines but as PICOs/clinical pathways per disease area often differ between countries, the topics would have to be carefully screened to ensure the submissions are suitable for all of the participating jurisdictions." (Adelaide Health Technology Assessment)

Table 95. Collaboration with international jurisdictions to deliver sustainable access to health technologies – impact on you/organisation by stakeholder type

	Very negative	Negative	Neutral	Positive	Very positive	Don't know	Sample size
Patient or consumer (or representative organisation)	0%	0%	0%	25%	58%	17%	12
Pharmaceutical / Medical technology company	61%	17%	11%	0%	0%	11%	18
University or research sector	0%	0%	20%	20%	60%	0%	5
Industry association / Peak body	25%	13%	0%	38%	13%	13%	8
Clinician (or representative organisation)	0%	0%	33%	0%	67%	0%	3
Consulting	0%	0%	0%	100%	0%	0%	1
State / Territory government	0%	0%	0%	100%	0%	0%	1

Other	0%	0%	33%	33%	0%	33%	3

Patients, Consumers and Representative Groups

There was support for this option from these stakeholder groups, with one group suggesting that leveraging international approvals could secure faster access for Australians. Another consumer group requested further information to ensure procurement issues were avoided later in the process.

"Australia is a relatively small, secondary market for many companies. There is often a delay in introducing technology here until it has been launched in larger markets like the United States and Europe. Therefore, Australia's assessment and approval process generally trails assessments conducted by the FDA (US), the European Medicines Agency (EMA – EU) and the Medicines and Healthcare products Regulatory Agency (MHRA – UK). There is an opportunity to streamline the approval process in Australia by providing a greater weighting to international approvals that have been granted by respected international agencies. International approvals could also be leveraged to secure faster access for Australians without compromising Australians when assessing updated or advanced models of already approved technology. For instance, Abbott Diabetes Care's original Freestyle Libre was superseded by the Libre 2 which features alarms when blood glucose levels deviated from a target range. In some markets this is now being replaced by Libre 3. In most instances, the updated models are simply an evolution of existing products." (The Australian Diabetes Alliance, Diabetes)

"There are not enough details to ascertain whether pooling demand with other countries to increase purchasing power would also cause procurement complications down the line." (Consumers Health Forum of Australia)

Pharmaceutical / Medical Technology Companies

The pharmaceutical companies expressed a strong negative response to this option, and they stressed that it would impede products launching in Australia. They stated they did not believe that Australia lacked purchasing power on its own and it would be counterintuitive to enter into buying blocks. They stated this option contradicted the Commonwealth's commitment to maintaining price confidentiality, a commitment they saw as critical to uphold.

"Novartis is supportive of the intent behind the option for HTA evaluation harmonization and international collaboration but has concern regarding how the differences in decision making policies and HTA guidelines will be overcome across jurisdictions without creating more work for each respective jurisdiction. Novartis is not supportive of international collaboration if the motivation is to "improve its (Australia's) ability to negotiate in relation to purchasing of innovative health technologies" (Options paper p. 168) as this is counter to the principles of HTA in that the price (or purchasing) of the relevant health technology is determined by the health outcome it delivers. It also puts at risk price confidentiality across jurisdictions which is critical for companies to maintain." (Novartis Australia)

"A buying block of multiple countries is not anticipated to improve access to medicines. This arrangement is predicated on a poorly justified assumption that Australia lacks purchasing power because of our relatively small population. However, the key challenge to overcome in achieving

access to medicines for Australian patients is not increased market share, but rather recognising fair value for the product. The proposed option presents additional barriers to access by putting confidential pricing arrangements and obligations at risk. These measures allow for solutions that deliver access for Australians. The expected impacts of block purchasing go against the shared goals of the Strategic Agreement and against improving HTA to better meet the needs of Australians." (Pfizer)

"(AbbVie strongly opposes this Option as it would impede products launching in Australia. The proposal is also in direct conflict with the Commonwealth's commitment to maintaining confidential pricing. The proposal to "form joint-common markets… to increase market share and purchasing power" does not represent a sustainable or viable option for manufacturers and would result in new health technologies not launching in Australia. The fact that Australian access to innovative medicines can depend on prices of certain innovative medicines being subject to special pricing arrangements is acknowledged by the Commonwealth in the latest Strategic Agreement, with current agreements and processes serving this purpose effectively. If the Australian government were to join a buying group with other markets, it is expected that manufacturers would need to waive rights to special pricing arrangements among the payers within the buying group to generate a common price. This would have detrimental international reference pricing implications that is expected to be unviable for manufacturers." (AbbVie)

"Roche is concerned that this recommendation will have the opposite effect to that which is intended. As acknowledged in the Options Paper, 'Australia is a small market within a global context'; Australia also has some of the lowest prices in the world compared to similar jurisdictions. If Australia were to join a buying group with other markets, it is expected that sponsors would need to waive rights to confidential pricing among the payers within the buying group to generate a common price. Consequently, this would have detrimental international reference pricing implications, of which the lowest/lower priced markets would lose out. It would ultimately result in new health technologies simply not coming to Australia. Alternatively, the price Australia would be required to pay which could be accepted by a sponsor would likely increase from the level which the Commonwealth has become accustomed to." (Roche Products)

Peak Bodies, Clinician/Researchers, Consultants, Government, Not-For-Profits (NFPs)

There was a mixed response from these groups: a number of them supported this option and believed that by forming partnerships with other countries Australia could negotiate favourable pricing, foster knowledge and improve affordability; while other groups reportedly believed the opposite – that it could potentially increase prices, due to pricing currently being set locally.

"Such an approach has been demonstrated by Beneluxa and others and may have benefits for Australia." (Adelaide Health Technology Assessment)

"As mentioned already in my submission - the companies that we are dealing with here as a country are international companies with very, very large capacities. It makes sense that countries have a uniform approach to them and to measuring, such as measuring carbon emissions." (The University of Notre Dame Australia)

"By forming partnerships with other countries, jurisdictions can negotiate favourable pricing agreements with manufacturers, enhance economies of scale, and improve affordability and

accessibility of essential health technologies. This collaboration fosters knowledge-exchange, promotes best practices in health technology assessment, and facilitates the adoption of innovative solutions to address shared healthcare challenges." (Society of Hospital Pharmacists of Australia)

"Conditions for pricing and purchasing are local. This is unlikely to produce better outcomes and low priced markets may be forced to accept higher prices." (Medical Technology Association of Australia)

Additional commentary about the Options Paper and HTA more broadly

There were a number of discussion points throughout the submissions that were not definitively within the Terms of Reference or directly in response to the proposals outlined in the Options Paper. These have been provided here for the Committee's reference.

There was some discussion from a handful of stakeholders about whether the HTA Options Paper is in alignment with the goals of the HTA Review and more broadly, whether they are in line with the National Medicines Policy.

One pharmaceutical company outlined that they do not support the proposals in the Options Paper for the following reasons:

"The NMP aims to achieve optimal health, social and economic outcomes for all Australians by fostering a highly supportive medicines policy environment. However, the current proposals outlined in the HTA Review Options Paper are at odds with the NMP's vision, potentially leading to unintended consequences and erecting new barriers to patient access. Further, the presentation of options that propose the introduction of cost containment measures not only extend beyond the HTA Review Scope but are not sustainable for the health technology industry." In their conclusion they State they *"cannot support the proposals outlined in the Options Paper. Whilst acknowledging the complexity of the HTA reform, it is imperative to ensure that any changes align with the overarching goals of NMP and prioritise the interests of patients and healthcare stakeholders across Australia"* (Gilead Sciences)

A medical technology company stated the *"HTA Review could have been used as an avenue to bold reform."* They highlight in their introduction that they believe Australia's approach to HTA *"fails to recognise expenditure associated with keeping people well and in the workforce as an investment"*. This company also comment that *"while there are parts of the Options Paper that we can support as having the potential to achieve faster access we are disappointed by the lack of alignment between the options proposed and the goals of the review, in particular the lack of bold reform to improve the value attributed to innovative medicines."* (Pathology Technology Australia)

Another consulting group outlined their concern about the lack of alignment with the peak body, the broader Commonwealth policies. (Consultant)

There was also comment and stated disappointment from MedTech companies about their stated lack of inclusion in the consideration process of the HTA Review (Medical Technology Association of Australia, Pathology Technology Australia)

There was also comment made by a patient representative group that drew attention to the reported effort required of these groups and that a perceived lack of funding for stakeholder participation may become a barrier to their ongoing participation as a result of the significant impact their involvement had on their volunteers (Collaborative Consumer Group Response).

Appendix A: Stakeholder Workshop Summary

The stakeholder workshops encompassed individual and group exercises, with opportunity to provide feedback and discussion on initial Options Paper reactions; health technology funding and assessment pathways; methods for HTA for Australian Government Subsidy (technical methods); increased transparency and improved access to HTA processes and outcomes; and closed with feedback and final comments. The face-to-face workshop included an addition exercise on increasing success rate of first pass applications and improving time to access (reducing number of resubmissions).

All workshops were attended by stakeholders representing many interest groups, such as patient advocates, patients with direct experience, non-affiliated individuals, clinicians, industry, researchers, peak bodies, evaluators and not-for-profit organisations. The Options Paper was considered too detailed for attendees to examine every reform option within the confines of the workshops, as such the exercises referenced above were designed to draw out key themes and issues from attendees. Encouragingly, many of the themes raised individually and as groups in the workshops were consistent with the findings summarised from the written submissions from stakeholders. Attendees were encouraged to use the written submission channels (questionnaire and email) to provide additional consultation relating to the potential options.

A summary of the key themes emerging from each exercise is provided below.

Exercise 1 – Initial Options Paper reactions

The following questions were posed to workshop attendees:

1. What are your initial reactions to the Options Paper – positive, negative or neutral?
2. What are the key positives of the proposed reform options and why?
3. What are your greatest concerns regarding these reform options and why?

Key themes that were raised in respect to attendees' overall responses to the reform options:

- Positive reception overall: Many stakeholders expressed a positive reaction to the Options Paper. The majority conveyed appreciation of the extensive work and consultation that have shaped the reform options presented.

  "Overall positive, it is an ambitious array of options that responds to the majority of points that were originally put forth by my organisation in Phase 1 of the consultation."

- Need for more detail: Several stakeholders expressed the need for more detail in the paper to understand the full implications of proposed reforms and any potential unintended outcomes. The term 'devil will be in the detail' was commonly heard across all workshops.

  "So, we responded neutral to a little bit positive. Mainly we feel like there's some great ambition there, but there is a general lack of clarity on what the outcomes will actually be, which makes us a bit cautious."

"Neutral – some great ambitions for reform, but a lack of detail in some areas that makes it difficult to be confident about what the change will look like and whether it will have the desired outcomes."

"I'm in general positive, but the main concerns that I had was that there were unclear timeframes of implementing some of these reforms once decided and also it seems to centre around a lot of things that we need to implement - it's unclear what sort of capacity we have in Australia, or the type of expertise that we have in terms of implementing some of these reforms."

- Concerns about complexity and length: Some stakeholders - especially those from consumer or patient advocacy roles - expressed they found the paper to be very dense and lengthy. As such viewed as potentially limiting the capacity of stakeholder to contribute to the discussion in an appropriately informed manner. It was noted that this complexity may make it difficult for all stakeholders to develop written submissions within the time constraints of the consultation period.

  "Recognise a lot of work and consultation has gone into this but my initial reaction was its 175 pages, and we don't have enough time. Very difficult for small, overloaded charities to engage, particularly with very complex processes where we may not fully understand the implications or potential unintended outcomes."

  "We felt that it was a really in depth look at everything, but also agree that the devil will be in the detail and the implementation of this. I read the paper a few times and my concern is also around the consultation period that we've got at the moment so that we can truly look into it at and having a post date of next week is a concern for some of us as well."

- Concerns about value recognition: Some stakeholders – most commonly those from industry - voiced that while the paper potentially supports timely access to medical technologies, some argue that it lacks significant reform options for better recognising what they perceive as "the full societal value" of new health technologies. If this value is not in turn reflected in the price paid, it was argued this may disincentivise product manufacturers and may either delay or prevent safe and effective treatments being launched in Australia.

  "We are concerned the initiatives don't go far enough and, in many cases, have already been subject to substantial consideration. We are concerned by the presence of savings measures and measures that seek to undermine the value of health technologies which we believe are not aligned with the objectives of the review."

- Negative reactions: A few stakeholders voiced negative reactions to the paper overall, and expressed concerns about a perceived undue focus on cost savings measures and measures that are viewed as potentially eroding capacity to bring new health technologies to the Australian market.

Key themes that were raised in respect to attendees' positive views of the Options Paper:

- Improving transparency of the HTA decision-making process: Many expressed they want to better understand how evidence presented in submissions is weighed and its ultimate impact on the committee's decision to recommend or not. This also extended to a call for what they perceive as sufficient guidance to stakeholders on what specific information needs to be included in submissions to be appropriately considered in the HTA process.

  "Improving transparency for consumers / clinicians. Improving publication of committee deliberations. Currently, it is difficult to have community buy in to the process - which might be seen as technocratic and secretive."

- Reduced time to access: Some stakeholders expressed that reforms streamlining HTA processes could lead to a reduction in access times.

  "A number of options present positive steps towards streamlining HTA process (e.g. proportionate assessment for cost min (although this should not have pricing policy attached and streamlining vaccine assessment) and could result in less time to access if implemented carefully and in consultation with all affected stakeholders."

- Greater proposed involvement of States and Territories: Stakeholders feel that HTA processes are currently hampered by a perceived lack of visibility of information available to the Commonwealth Government.

  "Greater involvement - possibly sharing information - with the States and Territories. Currently the HTA processes in the jurisdictions are hampered by a lack of visibility of information available to the Commonwealth Government."

- Consideration of broader value frameworks: The proposed inclusion of a broader qualitative values framework was commonly viewed as facilitating better decision-making, albeit with a caveat that there needs to be greater transparency on how such inputs are weighed and used in the decision-making process.

  "Qualitative value framework (provided independently managed) to better understand implicit considerations in decision-making - transparency and consistency."

- Risk-sharing pathways: Provisional pathways that permit earlier access to emerging but unproven technologies are welcomed but need safeguards both in terms of what specific outcomes could lead to defunding and also how patient expectations are managed through such a process.

  "Pathways that capture a sharing of the risk between industry and payer. Provisional pathways that permit earlier access, but also permit the government to build into the pathway the risk that the technology is not as effective as originally planned."

- Greater use of coverage with evidence development: Current data collection – especially in compiling real-world evidence and real-world outcomes - is viewed by many stakeholders as needing improvement. The reforms aimed at providing guidance and building system capacity to better capture such data is strongly supported.

  "Greater use of coverage with evidence development. This needs a huge amount of work. Current data collection is inadequate."

- Earlier Population, Intervention, Comparator, and Outcome (PICO) engagement: Stakeholders welcomed proposed options for early stakeholder engagement in the PICO scoping process to ensure the right inputs were selected for inclusion, albeit with some concern regarding the level of expertise needed to get the inputs to the model correct.

- Horizon scanning: The proposal to increase horizon scanning capacity was appreciated, especially for issues such as paediatric medicines.

  "I am quite interested in the section on futureproofing our systems and processes to proactively address some of the areas of unmet need, environmental considerations, and health technology assessments and also in the forward thinking on the capacity building in this space."

- Bridging funding: Introducing a bridging funding mechanism for therapies of likely highly added therapeutic value (HATV) in areas of high unmet clinical need (HUCN) is welcomed by many stakeholders to ensure patients are accessing often critically needed therapies in a timely manner.

- Greater First Nations involvement and consultation: Stakeholders universally supported greater equity among diverse communities including First Nations consumers.

  "Some of the specific positives that were mentioned was the opportunity to look at the HTA as a holistic pathway rather than looking at discrete issues, which has sort of been the approach to date. There were some really specific positives around the focused on First Nations peoples, proactive horizon scanning, consideration of equity and an emphasis on the inclusion of patient groups in the HTA process."

- Plain language summaries: Many stakeholders noted these offer scope to facilitate greater stakeholder and consumer understanding, engagement and input.

Key themes that were raised in respect to attendees' concerns relating to the Options Paper:

- Details around implementation of reforms: Stakeholders commonly expressed a degree of concern about a perceived lack of detail on how specific reform options will be implemented, especially the concept of a single HTA appraisal gateway and how this would be appropriately resourced.

  "Lack of clarity on some of the options and how the processes will be implemented - one HTA body for instance sounds good in theory but we are concerned that this will mean that we lose some of the fit-for-purpose functions of MSAC [Medical Services Advisory Committee] *that are currently working well for our consumer groups."*

  "There was a specific concern around whether the recommendation towards having a single committee and whether they would have the expertise and the capability to assess everything."

- Lack of value recognition: Many stakeholders indicated they believed the HTA Options Paper provided limited commitment to addressing their own value assessment concerns. It was argued that many of the options outlined would not result in improved value recognition of innovative therapies.

  "I think some of the components are probably a little bit too focused on some government savings as opposed to identifying what's best from an investment perspective in health technology in Australia and how we can improve access."

  "There were other areas which required further information around comparative selection, the discount rate and also how to work out a broader value with some general comments around the life-saving drug program and potentially at the minute the criteria is a bit too narrow because it's only focused on life-saving medicines, while there's some medicines that can have a significant impact on a patient's quality of life that aren't 'life-saving'."

- Patient access and affordability: Some stakeholders were worried that the reforms – if too tightly focused on cost reductions - might make it unaffordable or economically unviable for companies to list medicines in Australia, thereby reducing patient access and choice.

  "Concerned it will reduce patient access to treatments by making it unaffordable for companies to list medicines in Australia. Limits to resubmissions and mandated price reductions on streamlined processing are concerning."

- Concerns about cost-minimisation: Several stakeholders identified concerns about cost-minimisation linking to price reductions. There is a perception that this has not worked well in other markets, and risks Australia being deprioritised as a launch country for global manufacturers.

  "I think for me an area of concern is there could be a welfare loss or erosion of welfare through some of the options presented like the cost-minimisation minus approach and constraining negotiation options for highly added therapeutic value technologies as well."

- Public consultation process: A number of stakeholders expressed concern that they thought the public consultation process was being rushed, limiting the opportunity for many people to give feedback. They proposed further staged consultation over a longer period on each specific reform issue.

  "A key part of the suggested reforms deal with inclusion and transparency to better support consumer and other stakeholder input into the HTA process. However, there is a concern that this consultation may signal the level of sincerity embedded in these reforms given stakeholders are being asked to provide feedback on a 175 paper, with significant detail and covering many different issues in 4 weeks."

- Inaccessibility of Options Paper for consumers and some stakeholder groups: Several stakeholders felt the Options Paper itself was too overwhelming and dense a paper for consumers to understand & engage with.

  "The document itself is really overwhelming. It's very detailed, very technical, perhaps not fit or not suitable for all audiences to be able to sort of wade through the technical information, being able to provide input into the process concerns around the timing in terms of how long we have to make submissions to address a lot of the issues that are coming through."

- Consumer engagement and equity: While greater stakeholder engagement is welcomed, several stakeholders suggested this may likely come with an increased workload for consumer groups due to greater consumer engagement. There were concerns about equity, particularly regarding the free work done by the public to ensure the best HTA decisions.

  "There was, I think, concern and positivity for a potentially single unit HTA unit, but again the devil would be in the detail how that would be triaged, how there would be equity to consumers to actually have input into that."

Exercise 2 – Health technology funding and assessment pathways

The following questions were posed to workshop attendees:

1. Which of the reforms to funding and assessment pathways are you positive about and why?
2. Which of the reforms to funding and assessment pathways are you concerned about and why?

Key themes that were raised in respect to attendees' positive reactions to proposed appraisal pathway reforms:

- Proportionate appraisal and differentiated pathways based on risk: Stakeholders appreciate the concept of proportionate appraisal for streamlined pathways but expressed concerns about the triaging process. They indicated they worry that if decisions on level of uncertainty, HATV, and HUCN are made a paint seen as too early in the process (e.g. before full evaluation) this could lead to submissions being assigned to the wrong appraisal pathway.

  "We were generally in agreement that there was lots of positive intent to the funding and assessment pathways options. We like the idea of simplifying. We had lots of people that were interested and excited about the idea of bridging funding and having a new way of doing that, and the idea of proportionate assessment based on risk."

- Goal of timely and more equitable patient access: Stakeholders appreciated that the main objective is to remove delays in order for patients to access new health treatments in a timely manner.

  "Our group talked about how the goals relate back to shorter time to make a product available to patients and that's what all the stakeholders in this consultation want."

  "I think that it was encouraging that there were options for quicker access and more equitable access, particularly for areas of high unmet clinical need, and I think a call for equal responsibility between industry and taxpayers to ensure a fair share of that costing burden."

  "I think the proportionate appraisal is a good idea and perhaps drugs or technologies with overseas approvals and significant evidence can be fast-tracked, for want of a better term, to some degree. I think there's a broader appreciation that the processes take too long, so anything that can give us a faster process is important."

- Streamlined process for cost-minimisation submissions: Stakeholders see the potential in a streamlined process for cost-minimization submissions but are unclear about the level of evaluation these applications will go through. There is little support for any trade-off between final price achieved and a streamlined HTA process.

- Front loaded and empowered office of HTA: Some stakeholders expressed support for the proposal for a single entry point for HTA, which should provide oversight of the system, offer PICO guidance, triage applications to the appropriate pathway, and conduct horizon scanning.

  "A single gateway allows things to be more streamlined and the uniform unified process makes sense, also positive thinking around bridge funding - all of those have the potential to reduce the time that you know is perceived as being too long for approvals at the current stage. The fact that you know the assessment pathways being looked at was just seen as a positive."

  "We support the proposal (Option 2.1) for a single-entry point for HTA. It should provide oversight of the system, offer PICO guidance, triage applications down the appropriate pathway (as proposed in Option 2.1) and conduct horizon scanning (as proposed in Option 5.2)."

- New provisional listing pathway: The creation of an early resolution mechanism for submissions in areas of HUCN is seen as an important step, but stakeholders worry that without bridging funding, delays will likely be introduced as pricing considerations will take time to resolve. It was noted that drivers of the perceived under-utilisation of the existing managed access pathway (MAP) need to be considered if sponsors may be appropriately encouraged to utilise this pathway for bringing new technologies to the Australian market.

  "Creation of an early resolution mechanism for submissions in areas of HUCN is an important step but without bridging funding, delays will likely be introduced as pricing considerations will take time to resolve."

- Creation of therapy area specific pathways: Stakeholders see the creation of new pathways for vaccines and drugs for ultra-rare diseases as sensible and important for addressing delays in current HTA processes.

  "The major positives were the disease specific models which would allow for better understanding of health technologies and the other was the transparency."

Key themes that were raised in respect to attendees' concerns to proposed appraisal pathway reforms:

- Single HTA committee: Stakeholders see benefits in reducing duplication and streamlining the process, but also raise concerns about potential legislative implications and the importance of decision-making consistency.

  "A single HTA committee may see some benefits in reducing duplication and streamlining process, but some uncertainties that need to be considered, including any implications of legislation."

- Concerns over the triaging process: Stakeholders raised concerns around the triaging process and the high level of expertise needed early in the assessment process (with a view that that decisions in early stages have scope to significantly influence the final pathway and process).

  "There is a concern over the initial triage and then how that flows through the rest of the system because judgment calls are being made very early in the process before there's been a true evaluation of the submission and essentially it brings into question the level of expertise needed."

  "The 'risk' needs to be defined. Risk to patient? budget? I feel that this may already be intrinsically done (less though in approval/requirements for 'me-too' or 'more-of-the-same' therapies)."

- Early resolution with resubmission limits: A number of stakeholders expressed concerns about the perceived workability of the early resolution criteria and resubmission limits, and how these may actually hinder timely patient access.

  "The criteria for early resolution have the potential to not be workable and further consultation is needed to ensure they are sufficiently flexible but also still achieve the intended purpose of ensuring submissions aren't substantially delayed."

- Consolidation of pathways: Some stakeholders raised concerns about the practicality of consolidating pathways, citing issues such as the challenging nature of data review (across medicines, vaccines and other health technologies) by the Pharmaceutical Benefits Advisory Committee (PBAC) and potential resource constraints.

  "There's quite a degree of concern that we could get bogged down with a small group in HTA trying to manage a large amount of work coming through. So, slowing down the process is a concern, potentially a lack of understanding of the technology complexities. So, we see that as maybe an administrative burden, really time-consuming in committees.

It would be better if we have potentially had expertise-based groups to identify high unmet need medical technology for then recommending for fast tracking through HTA."

"We like the whole idea of consolidating all the pathways, especially for codependent technologies that will make things simpler for sponsors, but it may make life more complicated for people on the committee because they'll have to have that that requirement for broader expertise."

- Cost-minimisation: A number of stakeholders expressed concerns about proposals to accelerate funded access to new health technologies being conditional on cost-reduction, and not sufficiently considering additional patient benefits or outcomes.

  "The creation of a proportionate assessment pathway is an important step in removing unnecessary pressure on our HTA bodies. However, building in a price reduction (as proposed in Option 4.1) cannot be supported."

- Price policies associated with proportionate assessment of cost-minimisation: Some stakeholders express concerns that these policies may present further barriers to access by encouraging price erosion and limiting choice. Others suggested that price issues should be considered separately from the review entirely if the key objective is to improve timely access to new health technologies.

  "Cost-minimisation in itself isn't a negative thing. But there is not one proposal to accelerate funded access to new health technologies unequivocally. They all have conditions attached such as cost-reduction."

- Assessment of what constitutes HUCN (High Unmet Clinical Need): Several stakeholders noted the challenge in defining HUCN and concerns whether this would encompass more common diseases, not just rare illnesses with no available treatment.

  "What will the criteria be for HUCN? There are many rare diseases with no treatments and there is a risk that the identification of HUCN will be done in a way that prioritises larger cohorts (i.e. more common diseases). How can we build equity into this process?"

- Navigating bridging funding: Stakeholders noted the risk between government, sponsors and patients must be appropriately balanced. Lack of clarity around disinvestment also.

  "Bridging funding - how do we ensure that these do not open the window to politicise at a later date?"

  "There were just some concerns raised about bridging funding and that it could be too punitive for industry to meet the terms of the bridging funding and some concerns around the lack of detail and more work probably needed to be done in a co-design format for that to be successful."

Exercise 3 – Methods for HTA for Australian Government Subsidy (technical methods)

The following questions were posed to workshop attendees:

1. Which of the reforms to technical methods are you positive about and why?
2. Which of the reforms to technical methods are you concerned about and why?
3. Write down three ideas on how value for money assessment process for HTA can be most significantly improved.

Key themes that were raised in respect to attendees' positive reactions to proposed reforms to technical methods:

- Early stakeholder Input on PICO: Many stakeholders expressed positive sentiment about early input on PICO (Population, Intervention, Comparator, Outcome). They Stated that this will help develop a more aligned understanding of the therapy and key challenges.

  "We had a very positive response to the early stakeholder input to PICO, so that we can set the parameters early and stops things from changing across the process."

- However, some stakeholders comment that industry was not overtly listed in the stakeholder input section and that it was important that industry is included in these discussions.

  "The increase in stakeholder input to the PICO and yet industry members did call out industry being excluded. They would like to see and industry voice in that stakeholder group."

- Further, while early stakeholder input was welcomed by stakeholders, they expressed a need for reassurance this would not induce further delays in the assessment process.

  "Involvement of stakeholders in PICO selection is a positive outcome. Just need to make sure it's not a process that replicates what MSAC uses, which adds time rather than shortened time."

- Guidelines for real-world evidence (RWE) & non-traditional evidence: Stakeholders supported the implementation of guidelines for the use of RWE, including patient-reported outcome measures and other qualitative data but noted that they are light on details and reliant on appropriate resourcing and capacity building to ensure data is captured effectively.

  "Especially for rarer conditions, we clearly need to access real world evidence. We just need some thought about how that plays in what kinds of things are acceptable as real- world evidence and where they could or should be used."

- Genetic therapies and horizon-scanning: Some stakeholders noted the growing importance of genetic therapies and there being scope for significant efficiency gains if appraisal pathways could consider a broader range of applications (not just a narrow application based on the objectives of a single sponsor).

"The reforms around the role of genetics is very important. The system needs to be future proof so that efficiencies can be made in the system. We ultimately don't want people dying from lack of access to medicines/tests that have been approved in other indications. This is where horizon scanning would help as technology could be listed for a number of indications at the same time. The current process is too sponsor dependent where they are focused on the test for their drug only."

- Need for clarity on technical methods reform options: Some stakeholders expressed a need for greater detail on any potential changes to the technical methods before they can be appropriately considered in terms of positive or negative impacts.

  "There is overall a lack of detail on how the technical methods will change going forward and critically how they will be assessed by the PBAC. Given the importance of this it is difficult to determine which aspects are positive."

- Understanding how values framework inputs would be considered: While most support a greater emphasis on patient input to the assessment process, a number of stakeholders queried how such inputs would be weighed and used in the final recommendation process.

  "Value framework - but only if it ensures equity across patients and has some sort of weighting or ranking across the elements, so that submission sponsors understand what is important for future assessments."

Key themes that were raised in respect to attendees' concerns to proposed reforms to technical methods:

- Selection of the most appropriate comparator in PICO evaluation: Stakeholders expressed concern about the lack of reform proposed for selection of the comparator in the PICO model. They suggested that comparators should not always be the lowest cost option, but rather the technology that is considered best clinical practice in the treatment of a specific condition.

  "Quite often in in areas as was mentioned the rarer cancers and leukemias the appropriate comparators are not included to reflect medical practice in Australia."

  "The Options Paper does not provide any resolution to the issue of lowest cost comparator which is critical to resolving. Without reform there will continue to be an erosion of interventions being listed in Australia."

- Involvement of Stakeholders in PICO: Stakeholders questioned who the stakeholders will be invited into the PICO scoping process and how their input will be captured. Some stakeholders further questioned how consumers would be included, especially in terms of outcomes.

- Reform to the discount rate. Some stakeholders argued that the discount rate should be lower to bring Australia in line with comparable overseas markets & to ensure Australia remains attractive to those launching new health technologies.

"[The discount rate] is currently set at 5%, but internationally that's 1.5%. Australia's relatively high discount rate is disadvantage. Disadvantaging medicines which have a long-term therapeutic effect, things like vaccines."

- Determining value for money of health technologies: Stakeholders questioned how the value for money of health technologies will be estimated. They emphasised the need to consult a range of relevant data and expertise to truly address the value for money and the need for flexibility in the methods used to determine the value of a health technology.

  *"Some of the options presented around guidance for non-RCT [*non-randomised control trial] *evidence is reasonable but won't necessarily achieve the goal of faster access without commensurate value recognition. There's lots of information about what you need to present, but not necessarily how it would be more acceptable to the decision makers and the committee."*

- Updating guidelines for non-randomized and observational evidence: Stakeholders called for strengthening the updating of guidelines to ensure non-randomized and how observational evidence is weighted into HTA decision-making appropriately.

  "Real world evidence is great, but what is the weight of that?"

  "None of these things we're discussing are new and they're ideas that we've talked about for a long time for example: [in the report] real world evidence is discussed and then dismissed rather than taking the time to have a real deep dive or a wrestle into how we can use it and how it can be taken seriously."

- Inclusion of second order benefits: A number of stakeholders were concerned that the paper failed to engage with second order effects of new technologies from a valuation perspective. They argued that productivity benefits (e.g. where quality of life improves where a person can return to paid work or is at least less reliant on a carer) can be important benefits of new health technologies and should be better captured in value determinations.

  "Societal impact is not captured by the paper and whether HTA should now start to evaluate and address and accept societal impacts beyond the medicine added value."

  "And then finally, just it felt to me in the economic evaluation part of the paper that anything that led to higher prices was being shot down. So, whether that was comparator discount rate, second order effects were really disappointing with how these products need to be assessed in the context of the healthcare system when there are medicines like our company has a medicine for Alzheimer's, the value of that medicine sits in society."

- Requirement for technical expertise & resourcing: Stakeholders highlighted the need for technical expertise to evaluate the quality of non-RCT evidence; and who would pay for the additional resourcing needed to establish this type of capacity.

"The focus on real world evidence, but the guidance needs to be living because it's really moving at a rapid rate, and we really need to invest in skills and capacity of the system for real world evidence to make it actually work."

"It's great to see the desire to have better access and linkage of data and to use that to inform decisions. I guess on the flip side, it was, you know, who's going to pay for that?"

- Changes in guidelines and mindset shift: Stakeholders pointed out that changes in guidelines are not enough to drive more timely access to health technologies without a commensurate 'mindset shift'. Changes were said to be technical and not revolutionary, and they questioned whether delays in HTA would be addressed without broader reform being delivered. Further, stakeholders expressed even revised guidelines would need to be dynamic to keep up with rapidly evolving advances in health technologies themselves.

- Needs a more common thread on timing and implementation: Stakeholders expressed there was a lack of clarity on how additional and earlier consulting may change decision- making timeframes (e.g. is there a risk that bottlenecks are just moved up earlier in the revised process?). Some called for some worked examples or hypothetical case studies as to how a case would progress through the current HTA system vs. the proposed new process if all options were taken up.

Ideas that were raised in respect to how value for money assessment process for HTA can be most significantly improved:

Stakeholders in the face to face workshop were asked for their ideas on how value for money assessment process for HTA can be most significantly improved. They key theses from this exercise are presented below.

Defining value:

- Shift focus from viewing medicines funding as a 'cost' to an 'investment'.

- Societal perspective on value of medicines vs. other health spend & other spend across Commonwealth → where do taxpayers want money spent vs. where is it spent currently.

- Value should be linked to findings of National Medicines Policy (NMP).

- Right mix of stakeholders to judgments about value (including for special populations such as paediatrics).

What's data is included in value assessment:

Include secondary health benefits of new technologies:

  - Education
  - Welfare
  - Productivity

- Broaden assessment of value to include social value
- Environmental impacts embedded into CEA / CMA / CUA
- Generation of RWE (real-world evidence) /RWD (real-world data) | AW population
- Include patients (perhaps with assistance), consumers and clinicians in decision-making
- *Clear guidance on how each of these value elements can be measured (inc. through innovative evidence generation methods) and associated investment in improving data access infrastructure.*

Benchmarks of value assessment:

- Adoption of most plausible not most conservative parameters, based on broad stakeholder input.
- Improve comparative selection.
- Discount rates aligned with comparable markets. Discount rates equivalent to other HTA markets (e.g. UK & Canada) so it's comparable.

Transparency of value assessment:

- What has contributed to 'value' and how they have been weighted.
- Availability of data on meaningful outcomes.
- Increased trust in system.
- Get the societal perspective (e.g. a town hall or workshop) on the value of the medicine vs other government spend on health and non-health areas.

Other ideas:

- Fund an independent centre of excellent for methods development, and fund new studies of comparative effectiveness.
- Expand LSDP to ultra rare treatment with transformative value.
- Separate HTA from price negotiation i.e., independent HTA process.
- Managed entry schemes.

  Highly specialised therapies (i.e., chimeric antigen receptor T-cell therapy) to come from a single federal funding source.

Exercise 4 – Increasing success rate of first pass applications and improving time to access (reducing number of resubmissions)

The HTA reform Options Paper notes that a key goal is to improve time to access of innovative new therapies and reduce the number of re-submissions required before a therapy is listed on the PBS.

The following questions were posed to workshop attendees:

1. Are the proposed reforms likely to help achieve this goal? How come?
2. Are there any other specific changes you would make or examine to help achieve this goal / address the identified issues?
3. Are there any unintended outcomes from the options relating to the goal and what could be done to mitigate / reduce those?

Key themes that were raised in respect to attendees' comments on whether the reforms were likely to help achieve this goal:

- In summation there was no agreement among stakeholders that the reforms were likely to help achieve this goal and some stakeholders Stated that key performance indicators (KPIs) for HTA reforms and/or process would be required to achieve this goal.

- Success depends on the resources implemented and the willingness to invest – many felt that without additional resourcing it would be difficult to achieve reductions in time to approval.

- PICO scoping, resolution steps, and bridging funding were identified as helpful measures but requiring additional detail.

  "I think at face value a lot of the proposed reforms in mechanics of having early resolution, bridging funding etcetera will help."

  "It's a promising framework, but it's going to depend on the implementation (and specifically within that implementation the resourcing and what that might look to look like in the short term versus the longer or mid to long term). It will only be successful if there's a willingness to see health technology spend as an investment rather than a cost and a corresponding increased willingness to invest in innovation in Australia. That the piece around parallel processing remains challenging."

Key themes that were raised in respect to attendees' comments on suggested changes to achieve this goal:

- The process needs to be viewed as an investment, not a cost. A broader-based risk framework was suggested due to a perceived disconnect on the value of health technologies and how these are prioritised among broader government budget priorities.

- The need for clearer effectiveness measurement was commonly emphasized – KPIs need to be identified and tracked post the implementation of any reform.

"We had the need to decouple the budget impact from cost effectiveness assessments a little better so that pricing negotiations are perhaps a little less drawn out and protracted." "Policies, methods and corresponding KPIs should be with the lens of the national medicines policy [sic] *and there should be a sustained commitment to course correction over time."*

- An independent arbitrator was proposed.
- Some argued that there is a need for a better framework for determining medicines listed, better evaluation, and clear definition of timeframes.
- Suggestions include incentivizing speeding up applications, cooperation of HTA evaluation, and early input of expertise.
- Some stakeholders voiced their support for cooperation between TGA (Therapeutic Goods Administration) and HTA evaluations.

  "We noted that there is a lot more opportunity for interaction with the TGA [than HTA] before you submit your application. Then we were also talking about encouraging sponsors to bring medicines to the TGA so that you've got registration a lot quicker or aligned with other countries as well. It makes sense for the cooperation between TGA and HTA evaluation."

Key themes that were raised in respect to attendees' comments unintended outcomes of the reforms:

- Potential for system overload.
- If the goal is to accelerate submission, it may lead to the opposite effect as lower prices could make Australia a less attractive market for innovation.

  "We also discussed that in relation to pricing policies, if the goal is to accelerate regulatory submission, there could be an unintended opposite effect whereby Australia could move later in the global launch sequencing from 'wave one' to being a 'wave two' or 'wave three' market if the price is lower than Australia, and it becomes a less attractive destination for innovation."

- Submissions may be delayed, and process slowed down by people sitting on data for a long time before they submit (in order to get a better price).

Exercise 5 – Increased transparency and improved access to HTA processes and outcomes

The following questions were posed to workshop attendees:

1. Do you support the reform options put forward to achieve these goals? How come?
2. Are there parts of the process that are requiring more transparency than others? How could that be improved?
3. Are there any unintended outcomes from the options relating to the goal and what could be done to mitigate / reduce those?

Key themes that were raised in respect to attendees' support of the reform options put forward to achieve these goals:

- Support for transparency: Many stakeholders expressed support for the reform options, citing the importance of transparency in decision-making. They believe that making information accessible and easy to understand is crucial for all stakeholders. However, some stakeholders raised concerns about the potential conflict between transparency and commercial confidentiality.

  "There was really broad consensus that support for transparency is axiomatic."

  "Transparency is really important and there was also a concern that confidentiality should be maintained. Anything that is specifically held confidential should continue to be maintained, particularly around special pricing arrangements and around sharing of consumers data."

  "There was also the concern about transparency of the company's data and particularly how this can work when there's a global company, so they're not just dealing with Australia and concerned about sharing with other jurisdictions as well because the plans for Australia and New Zealand and Canada and the UK to have joint assessments."

- Stakeholder involvement: Stakeholders were generally supportive of increased involvement, particularly for consumers. They emphasized the need for genuine input and not just a 'tick box' exercise. However, some stakeholders expressed concern about over-representation by louder, better-resourced groups.

  "The more information they have the better they can decide what is best for them, but what medium will be used? The document is pretty technical, could there be a consumer document that sits alongside it?"

  "There's obviously some patient groups where they are really well engaged in in the HTA process and have you some really positive influence on that on that process. But there's obviously many other patient groups who just don't have 111 members as part of that association and they don't have the resources to engage in that process."

- Inclusion of diverse communities: Stakeholders appreciated the inclusion of First Nations people and also called for the explicit inclusion of Culturally and Linguistically Diverse (CALD) and non-verbal communities.

  "Should not just be First Nations, but there are other various minority and vulnerable populations should have like a clear and transparent input into the process."

  "Other populations that have might have multiple barriers to engagement and needing additional support for that."

  "There are underserved populations, certain groups that perhaps their needs won't be met with these. One example was pediatric patients."

- Consumer engagement: Stakeholders strongly supported consumer, clinician, and other stakeholder engagement and consideration in the HTA, earlier in the process. They emphasized the importance of this step, especially for rare diseases that may have no cure and very few effective treatment options.

  "At the moment the focus seems to be around the patient groups. But what about the patients? Individual consumers and patients who with lived experience their families, how do we how do we engage those people and capture their voices?"

  "Early Statehood stakeholder input on PICO would really help, provided there's the right support there for patients to appropriately engage with that process."

Key themes that were raised in respect to attendees' Statements on parts of the process that are requiring more transparency than others:

- Stakeholder input and decision-making: Stakeholders commonly expressed a desire for more transparency regarding the influence of their input on final decision-making. They suggested that if their input is used, they should understand how it impacts decisions. Similarly, if it is not used, why that is the case.

- Reimbursement decisions: There was a call for more transparency around how reimbursement decisions are made and what information is considered.

  "Greater clarity about how different aspects of decision-making were weighted. For example, how much weighting's given to patient input in terms of HTA committee decision-making and greater transparency for consumers about why things aren't available in Australia? Or why drug might be available in another country but not here?"

- Industry as a stakeholder: Stakeholders expressed a desire for the industry to be recognized as an important contributor to the process and methods. While appreciating the needs of industry to be appropriately weighed against those of patients and the broader community, it was suggested a 'more mature' relationship with industry could assist in the broader goal of more timely access to health technologies.

  "We need to find ways to overcome the barrier of commercial and confidence, and that is often used as a weapon to exclude the patients and patient groups and perhaps they could be signing non-disclosure agreements."

- Decision-making criteria: There were questions about the criteria used for decision- making – not only in terms of specific decisions, but how the HTA's thinking more broadly may be evolving in line with evolving technologies and evidence.

  "Transparency through the various steps in the process where it might not be until the communique comes out from essentially one of the higher-level decision makers that we get insight into what some of the advisory groups that sit below might be saying."

- Evidence Requirements: Stakeholders mentioned the need for agreed definitions of words being used and clarity on specific evidence requirements.

- State government involvement: The idea of involving State governments was seen as potentially beneficial, but there were concerns about this further complicating (and potentially delaying) the process, as well as differing needs among individual States & Territories.

  "The need to be more communicative with State governments, particularly around some of the shared funding models and the extent to which some of the inherent challenges of the Federated system make that bureaucratic and a bit of a pain in the neck."

  "Particularly when it comes to interactions with the States and Territories. It's acknowledged that there should be collaboration there, considering the decisions that are made can have big impacts on the State system and they also need to be ready to implement decisions."

- Consistency across communications: It was mentioned that inconsistencies existed in how recommendations from PBAC and MSAC were currently presented, and that specifically PBAC Statements could be released at time when stakeholders have greater capacity to effectively engage and respond.

  "Recommendations from PBAC and MSAC are not exactly presented in the same way, and it'd be good to get some consistency around this."

  "The outcomes Statements by the PBAC come on a Friday night at 17:00 and leaves consumers and stakeholders scrambling around on the weekend to make something of what the results mean, it's very difficult for industry to manage that and has a detrimental effect to consumers."

  "The FDA is a working example has the time clock option and I know that it States the website and a real tracking in real time is all very helpful."

Key themes that were raised in respect to attendees' Statements on unintended outcomes from the options relating to the goal:

- Complexity and delays: Stakeholders expressed concern about the potential for overly complex or cumbersome processes leading to delays. Some argued that Australia consistently negotiates lower prices than comparable markets, leading to an ongoing focus on including Australia as a reference country for international reference pricing. They emphasized that full transparency of all information included in HTA evaluation is not required for stakeholders to meaningfully input into the process.

- Resource impact: The need to avoid proposals that result in a significant impact on resourcing or are too onerous was another key point raised by stakeholders. They highlighted the potential resource intensity of the proposed changes and questioned how to word a PSD to be understandable and unbiased.

  "It's a big onus on the groups to put these this feedback together, so they need to reduce waste and be time efficient."

  "How to word a PSD to be understandable and unbiased?"

- Communication and levels of health literacy: Stakeholders noted that plain language summaries may fail to communicate the complexity of decision-making processes, including uncertainty. They also pointed out that transparency and improved communication rely on a certain level of health literacy. It was suggested there is a risk of increasing inequities unless there is an accompanying effort to improve health literacy across affected consumer groups.

  "When conveying decisions to patients (the public), much of the information is very technical (in PSDs) which can be difficult for patients to grasp. NICE seems to have separate plain language Statements for this but unsure how this has been received by the public. We need to ensure what we have implemented be re-evaluated to see what needs to be improved or if it continues to be needed."

Closing comments

Stakeholders were asked for their final comments prior to the end of the workshops.

Key themes that were raised in attendees' final comments:

- Timely access to treatments: Stakeholders emphasized the importance of providing Australians with timely access to the best treatments available. They suggested that reforms should focus on improving the time to approval for new technologies and ensuring timely and equitable access to health technologies.

  "The most important thing is that we see bold reform in implementing and then delivering on clear metrics that respond to the goals of the review (improved time to access and maintaining Australia as a first-wave launch country)."

  "There was a lot around financial impact, you know risk to budgets being unsustainable, all that sort of information as well and I think when we start to let that be our main

consideration, we will lose lives. How do we bring the best outcomes and our health system to everybody and not let it always just be about finance?"

- Transparency in decision-making: Increased transparency in decision-making processes was another key point raised by stakeholders. They called for transparency and accountability in how decisions are made, as well as more rigorous in-market assessment to ensure funded treatments are delivering their intended outcomes.

- Stakeholder engagement and collaboration: The need for increased stakeholder engagement and collaboration was also emphasized. Stakeholders called for a commitment from the government to implement a full package of reforms, and to co- design all elements with stakeholders.

  "Improved relationships between Government, Industry and those involved in the pathways. This permits a level of pragmatism that is required - having highly specific rules might be preferred by Industry or Government, but it reduces flexibility."

- Consideration of broader Impacts: Stakeholders suggested that the reforms should take a wider view of value, considering health outcomes, financial impacts, and environmental impact. They also highlighted the need for the HTA process to be future-proof for medical devices and digital health technologies, and for the evaluation of new technologies to include the infrastructure and ancillary costs associated with setting up a clinical service.

  "The paper is silent on health technologies used in combination, and we think that's an area where the clarity is required. Oncology is probably a common example you'll have two separate health technologies which we use at the same time and our system isn't good at determining the relative value of the components of that combination, and this is one of those areas of horizon scanning where we know it's more increasing. And we don't have clear pathways for doing that combination assessment of two health technologies to be used for a single purpose."

  "Our business has been using what's called a triple bottom line approach, which considers economic, environmental and social impacts and I think we should take a similar kind of lens to HTA considerations and think about an adapted triple bottom line which considers health outcomes, which it always has financial impacts, which it always has, but also now to add the environmental impacts and that could really run throughout all of the sections of the document."

Appendix B: Written Submissions

The HTA Review Committee appreciates the time and effort made by stakeholders who continued to this consultation round though a written submission. All submissions received were duly considered by the committee in the development of its final recommendations to Government.

In total, some 132 written submissions were received across the online survey and those emailed directly to the HTA Secretariat (from a total of 126 organisations, companies, or individuals). Quotations included is this report are drawn from the following submissions where the organisation or companies agreed for their submission to be published or quoted from (excluding those received from individuals, whose names are not published below):

- A.Menarini Australia
- AbbVie
- AccessCR Pty Ltd
- Adelaide Health Technology Assessment (AHTA), University of Adelaide
- Alexion
- Amgen
- Antengene Australia
- Asthma Australia
- AstraZeneca
- Australian Antimicrobial Resistance Network (AAMRNet)
- ausEE Inc.
- Australasian College of Emergency Medicine
- Australasian Leukaemia & Lymphoma Group and Haematology Society of Australian & New Zealand
- Australian Centre of Accelerating Diabetes Innovations (ACADI)
- Australian Healthcare and Hospitals Association
- Australian Patient Advocacy Alliance
- Australian Patients Association (APA)
- Bayer Pharmaceuticals ANZ
- Biogen
- BiomeBank
- Boehringer Ingelheim
- Brain Foundation
- Breast Cancer Network Australia
- Bristol Myers Squibb Australia
- Cancer Council, CNSA, COSA, PCPA, MOGA
- Cell and Gene Catalyst (AusBiotech)

- Centre for Sustainable Medicine, Yong Loo Lin School of Medicine, National University of Singapore
- Childhood Dementia Initiative
- Christopher Steer (Individual clinician)
- Climate and Health Alliance
- Consumers Health Forum of Australia
- Crohn's & Colitis Australia
- CSL Limited
- Deakin University
- Dementia Australia
- Dragon Claw Charity
- Doctors for the Environment Australia
- Eli Lilly Australia
- Genetic Support Network of Victoria
- Gilead Sciences
- GSK
- Haemophilia Foundation Australia
- Health Services Research Association of Australia and New Zealand
- Healthy Environments and Lives NHMRC national network
- Illumina
- Immunisation Coalition
- Ipsen
- IQVIA
- Johnson and Johnson Innovative Medicines
- Leukaemia Foundation
- LSDP Expert Panel
- Lung Foundation Australia
- Maimon Research
- Medical Technology Association of Australia
- Melanoma & Skin Cancer Advocacy Network (MSCAN)
- Menzies Institute for Medical Research, University of Tasmania
- Metabolic Dietary Disorders Association
- Mito Foundation
- MND Australia
- Monash Children's Hospital
- MSD Australia
- MTPConnect / Australian AMR Network
- National Aboriginal Community Controlled Health Organisation (NACCHO)

- National Blood Authority
- NeuroEndocrine Cancer Australia
- Neurological Alliance Australia
- Novartis Pharmaceuticals Australia
- Omico
- Ovarian Cancer Australia
- Painaustralia
- Pathology Technology Australia
- Pfizer
- Pharmacy Guild
- PRIMCAT Consumer Panel (Independent Consumer Panel)
- Public Health Association Australia
- Royal Australian and New Zealand College of Ophthalmologists (RANZCO)
- Rare Voices Australia
- Royal College of Pathologists of Australia (RCPA)
- Roche Products
- Royal Australasian College of Surgeons
- Royal Australian College of General Practitioners
- Servier Laboratories (Aust) Pty Ltd
- Society of Hospital Pharmacists of Australia
- Sydney School of Public Health and NHMRC Clinical Trials Centre, Faculty of Medicine and Health, The University of Sydney
- Takeda
- The Australian Diabetes Alliance
- THEMA Consulting
- UCB Australia
- University of Melbourne
- University of Notre Dame Australia
- West Australian Department of Helath
- Wiser Healthcare

Please note the 'Collaborative Consumer Group Response' was endorsed by the following organisations:

- Arthritis Australia
- ausEE Inc.
- Australia Pompe Association
- Australia Sickle Cell Advocacy
- Australian Patient Advocacy Alliance (APAA)
- Bowel Cancer Australia
- Breast Cancer Network Australia
- Cancer Voices Australia
- Canteen Australia
- Childhood Dementia Initiative
- Crohn's & Colitis Australia
- Cystic Fibrosis Australia
- Dementia Australia
- Dragon Claw
- Eczema Support Australia
- Emerge Australia
- Epilepsy Australia
- Epilepsy Foundation
- Haemophilia Foundation Australia
- Head & Neck Cancer Australia
- Hearts4Heart
- Huntington's Australia
- Leukodystrophy Australia
- Lived Experience Australia
- Liver Foundation
- Lung Foundation Australia
- Lymphoma Australia
- Metabolic Dietary Disorders Association (MDDA)
- Mito Foundation
- MJD Foundation
- MND Australia
- MS Australia
- Muscular Dystrophy Australia
- Myasthenia Alliance Australia
- Myeloma Australia

- National Aboriginal Community Controlled Health Organisation (NACCHO)
- NeroEndocrine Cancer Australia
- Neurological Alliance Australia
- Ovarian Cancer Australia
- Pancare Foundation
- Parental Nutrition Down Under (PNDU)
- Parkinson's Australia
- Patient Voice Initiative
- Primary Ciliary Dyskinesia (PCD) Australia
- Prostate Cancer Foundation of Australia
- Rare Voices Australia
- Save Our Sons Duchenne Foundation
- SCN2A
- WMozzies
- XLH Australia

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