

Report of the Health Technology Assessment Policy and Methods Review Reference Committee

January 2024

Health Technology Assessment

Policy and Methods Review

Consultation options paper

This is a report of the [HTA Review Reference Committee](https://www.health.gov.au/committees-and-groups/hta-review-reference-committee), which is overseeing the Health Technology Assessment Policy and Methods Review. Statements using terms such as ‘we’ are referring to the Reference Committee.

# Overview

The Reference Committee for the Health Technology Assessment Policy and Methods Review (HTA Review) has received extensive inputs in relation to Health Technology Assessment (HTA) systems in Australia and overseas – how they are performing, and how well they are serving the needs of Australians.

This paper presents options for reform being considered by the Reference Committee to improve Australia’s HTA policies and methods and the funding and approval pathways.

The aim of this paper is to facilitate stakeholder feedback on the potential options for reform relating 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.

The Reference Committee will consider the feedback received through this consultation, alongside other inputs, before deciding on final recommendations and delivering its final report.

The Reference Committee has undertaken a holistic, systemwide review of Australia’s HTA system, in line with the [terms of reference](https://www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-terms-of-reference) for the HTA Review. As a result, many of the issues identified and options developed incorporate multiple elements and features across the HTA system and the topics within this paper may be relevant to multiple heading areas. The document will cross-link where possible, and an

Index can be used to search for specific topics.

The paper is broken up into the following chapters:

* [Transparency, communication and](#_Transparency,_communication,_and) s[takeholder involvement in HTA](#_Stakeholder_involvement_in)
* [Health technology funding and assessment pathways](#_Health_technology_funding)
* [Methods for HTA for Australian government subsidy (technical methods)](#_Methods_for_HTA)
* [Health technology funding and purchasing mechanisms and decisions](#_Health_Technology_Funding_1)
* [Futureproofing our systems and processes](#_Futureproofing_our_systems)

Under each of the headings, the report set outs:

* the current state in Australia and other jurisdictions (where applicable)
* what was heard from stakeholders
* identification or understanding of the issues (where these are formed), and
* potential options for reform.

This options paper should be read in conjunction with the following reports commissioned to inform the HTA Review:

* [International Health Technology Market Approval, Funding and Assessment Pathways](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-review-international-health-technology-market-approval-funding-and-assessment-pathways?language=en) (Paper 1)
* [Horizon Scanning and Early Assessment](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-horizon-scanning-and-early-assessment?language=en) (Paper 2)
* [Determination of the Population, Intervention Comparator, and Outcome (PICO)](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-determination-of-the-population-intervention-comparator-and-outcome-pico?language=en) (Paper 3)
* [Clinical Evaluation Methods in HTA](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf) (Paper 4)
* [HTA Methods: Economic Evaluation](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-hta-methods-economic-evaluation?language=en) (Paper 5)
* [Funding and Purchasing Decisions and Managing Uncertainty](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-funding-and-purchasing-decisions-and-managing-uncertainty?language=en) (Paper 6)
* [Optimising the Availability and Use of Real-World Data and Real-World Evidence to Support Health Technology Assessment in Australia](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-optimised-real-world-evidence-to-support-health-technology-assessment-in-australia?language=en) (Paper 7)
* [Australian market approval, funding and assessment pathways and timeframes (Paper 8)](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-australian-market-authorisation-funding-and-assessment-pathways-and-timelines?language=en)

Excerpts from these reports have been used as part of the content of this paper. Where larger bodies of text from these reports form part of the content to be considered under various sections of this paper, hyperlinks will be used.

The options presented in this paper do not represent the Reference Committee’s final views on the merits of any option. However, they provide an indication about what is considered the best direction for reform. The summary table below sets out these key options.

|  |
| --- |
| ***A note on terminology…***  Australia uses the term 'consumer' to refer to 'patients, their families/carers, consumer organisations'. However, the academic literature refers to the same people as 'patients' or 'patients, carers and patient organisations' reflecting the dominance of the term in international practice (Australia, Singapore and New Zealand being the exceptions). |

# How to provide input

A survey will be opened shortly seeking feedback on the options presented in this paper. This document and associated webpages will be updated to reflect further consultation information once available.

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# Preface

All individuals and organisations that have participated in the HTA Review are keen for Australians to be able to access the latest breakthrough health technologies as quickly as is possible, and in line with the [National Medicines Policy (NMP)](https://www.health.gov.au/resources/publications/national-medicines-policy?language=en), at an affordable cost.

When deciding whether a health technology should be funded or subsidised, the fundamental questions that need to be asked are:

* Does it work?
* Will it work as well as, or better than, what Australians already have access to?
* Will Australians be better off overall if it is funded?

Australia’s HTA policy and methods provide the framework for how these assessments are to be made. They set out how safety and efficacy of health technologies are measured, how they compare with alternative therapies, how their value for money is estimated, how appraisals may be modified for equity and other policy considerations, and how confident the HTA Committee can be in the evidence they have reviewed to support the HTA decisions/advice.

As the Reference Committee for the HTA Review, we have observed that HTA arrangements are complex to navigate for most stakeholders and that this complexity has increased over time through many reforms developed jointly by industry and government.

Some of this complexity is necessary. Human health and the practice of medicine and healthcare are complicated and health technologies addressing areas of unmet need are increasingly innovative.

Good decisions need to account for the complexity of these areas. Nevertheless, we have also seen that several aspects of the HTA and funding system could be simpler.

#### What have we heard?

Throughout the HTA Review we have heard from many individuals and organisations. Largely, stakeholders report that Australia has a world class HTA system that works well in many aspects to provide subsidised access to therapies to patients, with many stakeholders acknowledging the dedicated individuals and teams throughout the system as a major contributor the success of the system. We have also heard from stakeholders that they are concerned about how consumers, patients, First Nations peoples and others are included and considered in HTA processes and decision-making. We heard concerns about how different types of evidence are considered and weighted, how HTA advisory committees value health technologies, and how long it takes for them to be funded and made broadly accessible to Australians.

Throughout the HTA Review, we have thoroughly considered the extensive input and evidence presented through a range of channels including:

* the [first round of public consultations](https://ohta-consultations.health.gov.au/ohta/hta-review-consultation1/) for the HTA Review
* deep dive discussions with individuals and organisations including patients, clinicians, representative organisations, industry and international experts
* submissions to, and the report and recommendations of, the House of Representatives Standing Committee Inquiry ([the Inquiry](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report)) report, [The New Frontier - Delivering better health for all Australians](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report) (The New Frontier report)
* Other relevant Committee inquiry reports such as that into [juvenile arthritis](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/rheumaticdiseases)
* the Government’s responses to these reports.

Additionally, we have thoroughly considered [commissioned reports prepared by experts in HTA](https://www.health.gov.au/resources/collections/health-technology-assessment-policy-and-methods-review-research-and-analysis-papers) analysing contemporary research, relevant methodologies and purchasing practices used by comparable international jurisdictions, and their applicability to the Australian context.

#### What issues have we identified?

We have observed that Australia’s processes and how they are used in practice mean that, in many circumstances, health technologies are not funded in the shortest possible time. Companies that supply health technologies rarely make applications for funding at the earliest opportunity; they are frequently not acceptable in the first instance, and for some technologies, the pathway has more steps and is more complicated than it needs to be.

Globally, there are issues relating to limited commercial incentives to develop certain types of products, such as new antimicrobials, which contribute to the global issue of antimicrobial resistance (AMR). AMR is an exceptionally complex multifaceted issue, clinically and economically, and is being impacted by the current HTA and purchasing practices both in Australia and globally.

We have identified that, while evidence sources beyond traditional clinical trial-based evidence of clinical efficacy, safety and cost effectiveness may be considered and factored into decision making, the current guidance (such as the PBAC guidelines), public summaries and other communications do not make is clear how to include this input, how they are used by and to what extent they modify the advice of, the HTA committee.

Lack of transparency and clarity is an overarching theme impacting the performance of many elements across the whole HTA system. Stakeholders such as patients and clinicians have unequal knowledge of the application, the evidence submitted with it, and the process for its assessment. We have identified that while there is a vast amount of information available, the communication of this information is often not fir-for-purpose leading to a lack of transparency. The review process has revealed many misconceptions relating to HTA systems, processes, methods, and decisions which is a symptom of the lack of transparency including fit-for-purpose methods of communication. Relatedly, we consider that involvement of patients, consumers, clinicians, First Nations peoples and does not occur early and regularly enough through the HTA continuum to realise the full potential value of their involvement.

We identified that Australia’s systems for funding health technologies do not look proactively at the unmet needs in Australia and proactively seek possible health technologies to address them. As with most other international jurisdictions, Australia’s system is reactive in that it relies on companies that supply health technologies to make submissions for subsidy, and there is no mechanism to proactively seek submissions. Similarly, Australia does not systematically scan what health technologies are available, or imminently available elsewhere in the world that might address needs in Australia better than the currently available options.

Health technologies may not perform as well in the real-world as they did in trials for a range of reasons. It was identified that Australia does not have sufficient systems to evaluate whether subsidised health technologies work as well as expected after the original subsidy assessment and do not routinely or systematically measure how well health technologies that have been funded perform compared to alternatives – whether patients and the Australian community are better off from accessing the health technologies that have been funded.

It noted that there is some collection of data and analysis to measure the performance of health technologies in different jurisdictions and for specific products, such as those funded under provisional approval or interim arrangements, and those selected for post market reviews. However, the quality and integration of this information is not fit-for-purpose to inform decision-making. Further, improvements must be made to the data capture, quality, and availability to measure the performance of the HTA systems and processes, including transparency of different stages and their responsible actors within the system.

#### What potential solutions have been considered?

We have considered that there are opportunities to reduce complexity, better engage with those who are impacted by HTA decisions, improve system performance, ensure Australia’s health technology funding programs are meeting the health needs of Australians to the greatest extent possible, and be confident that Australians are getting the benefits expected when the Australian government decides to fund health technologies.

This paper sets out a range of options for reform that have been developed based on review, consideration, and synthesis of the extensive input we have received throughout the HTA Review process.

These options span the lifecycle of health technologies, from development, through initial funding decisions to assessment of their performance after they are funded. They include options for reform to how patients, consumers and healthcare professionals are engaged in HTA, as well as to the technical methods used to estimate benefits and costs of new technologies compared to existing available healthcare, and measuring how effective and safe they actually are after they have been funded and made widely available to the Australian community.

# Executive Summary

The HTA Review is one of the key commitments in [the Strategic Agreement between the Commonwealth and Medicines Australia](https://www.pbs.gov.au/info/general/medicines-industry-strategic-agreement). Its primary objective is to ensure that Australia’s assessment processes keep pace with the rapid advances in health technology and minimise barriers to access.

The [terms of reference](https://www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-terms-of-reference) for the HTA Review were published on 22 March 2023. Since then, the Reference Committee has traversed extensive inputs in relation to Health Technology Assessment systems in Australia and overseas – how they are performing, and how well they are serving the needs of Australians.

A broad range of stakeholders have provided valuable insights through various consultations, including [Consultation 1](https://ohta-consultations.health.gov.au/ohta/hta-review-consultation1/), which received more than 110 submissions, and deep dives with the Reference Committee which covered a range of topics. Stakeholder input was bolstered by the 7 [research and analysis papers](https://www.health.gov.au/resources/collections/health-technology-assessment-policy-and-methods-review-research-and-analysis-papers) developed by experts in HTA, analysing current methods, contemporary research, relevant methodologies, and purchasing practices used in HTA in Australia and internationally.

This paper has been developed to facilitate further stakeholder input through Consultation 2 on potential options for reform that have been proposed through the HTA Review or developed by the Reference Committee through its consideration of the input received to date.

### Goal of options for reform

The Reference Committee has identified several areas where Health Technology Assessment and funding approaches could be improved to better meet the needs of Australians.

The options the Reference Committee presents in this paper are those that it has identified could address the issues raised and support the objectives set out in the [terms of reference for the HTA Review](https://www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-terms-of-reference?language=en). That is:

1. deliver Australians equitable, timely, safe and affordable access to a high-quality and reliable supply of medicines for all Australians
2. adopt a person-centred approach in HTA
3. deliver the outcomes sought by recommendations from [the Inquiry](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report) that are agreed in principle in the [Government Response](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Government_Response)
4. further the objectives of the new [NMP](https://www.health.gov.au/resources/publications/national-medicines-policy?language=en)
5. ensure HTA policy and methods are well adapted to and capable of assessing new technologies that are emerging or are expected to emerge in the coming years.

While making sure they are:

1. implementable and sustainable for both health funders (Commonwealth, state, and territory) and the health technology industry
2. do not compromise assessment of patient safety, effectiveness and cost, or advice to Government on subsidy of health technologies.

### How long HTA processes take in Australia

The Reference committee considered several analyses on how long HTA processes take in Australia compared to other countries and the reasons that they may not happen in the minimum possible timeframe.

#### Medicines

The Reference Committee saw that the shortest possible timeframe for funding medicines in Australia was fast compared to most other countries but that medicines were rarely funded in the shortest possible timeframe. Parallel processing and a 17‑week HTA cycle for medicine make it possible for medicines to be funded quickly, but delay in submissions being made, repeated resubmissions and extended price negotiations in some instances prevent this from happening.

Overall, Australia is reported to be in the middle of Organisation for Economic Co-operation and Development (OECD) countries in terms of the actual time it takes for a medicine to be funded.

#### Other health technologies

The Reference Committee found that the minimum possible timeframe was significantly longer for vaccines, advanced therapies (including high cost Highly Specialised Therapies (HSTs) funded through the NHRA), and medicines funded through the Life Saving Drugs Program (LSDP). This was due to the additional processes in the assessment and funding decisions for these products.

### Transparency, communication, and stakeholder involvement in HTA

#### Transparency and communication of HTA pathways, processes and decisions

Communication, clarity, and transparency were overarching themes raised across all stakeholder groups, affecting the performance of many aspects of the HTA system. The Reference Committee considered that while a lot of information is available regarding HTA systems, processes, pathways, and decisions, it remained challenging for those impacted by HTA decisions to find and understand the information they were looking for. This is due to documents not being written in accessible language, or specific information is not published. Many of the published documents are highly complex and technical, prepared for different audiences and purposes, and are presented on multiple websites. Additionally, there is a need for meaningful, timely information on HTA submissions and the factors influencing HTA decisions.

A suite of reform options to improve the performance of the HTA system through enhancements to transparency and communication have been proposed, including the timely provision of plain language summaries of various documents (e.g. HTA submissions lodged by sponsors (usually the companies that supply the health technologies), committee decisions, and HTA guidance material). Further, options to improve the HTA webpages, including development of a data-driven dashboard and the communication of currently available information, will make the HTA system, its processes and performance, easier to understand and navigate for all stakeholder groups. Importantly, these options complement those detailed under the Stakeholder involvement in HTA section.

#### Consumer, clinician and other stakeholder involvement and consideration in HTA

The main mechanism for stakeholders to be involved in HTA in Australia is though the opportunity to provide comments on submissions made to advisory committees.

The Reference Committee noted support from stakeholders for processes that allow consumers, patients and clinicians to engage in HTA. However, there was general concern raised that it was unclear how input was used. Stakeholders also expressed a view that input should be sought earlier in the HTA process. This paper presents options to develop an engagement framework and strengthen consumer evidence that may help to address these issues.

#### First Nations peoples involvement and consideration in HTA

The Reference Committee considered that while there are some mechanisms to promote equity in access to medicines for First Nations peoples, a lack of formal and routine involvement of a First Nations health representative and consideration of First Nations health outcomes is contributing to health inequity. Stakeholders purported that several medicines integral to the health and wellbeing of First Nations peoples are not listed on the PBS, and data revealed disparity in access to medicines for First Nations peoples with significantly lower expenditure on medicines for this population.

The Reference Committee considers that HTA advisory committees would benefit from more formal arrangements for ensuring consideration of impacts on First Nations peoples in relation to all funding (listing and delisting) decisions, including a proactive approach to identifying unmet needs and potential therapies to meet these needs.

#### State and territory governments

The Reference Committee considers that there are significant opportunities to increase collaboration with state and territory governments in HTA processes associated with federally, and jointly-funded health technologies, including increased opportunities for input, consultation and work sharing in relation to horizon scanning, HTA evaluations and data-sharing.

### Health technology funding and assessment pathways

Currently, funding mechanisms in Australia for different health technologies are generally based on the type of health technology (e.g. medicines, diagnostic tests, blood products) and, in some cases, the setting where the health technology will be delivered (e.g. inpatient or outpatient setting). At present, the funding program for a health technology primarily determines the HTA pathway that will be used to evaluate that technology. The existing separation of pathways based on technology type is historical, and becoming increasingly complex to navigate as new health technologies challenge the distinctions between different technology types.

The Reference Committee does not consider that development of further assessment pathways should be created. The committee considers it beyond the scope of this review to recommend wholesale consolidation of health technology funding in Australia. However, it considers that the assessment functions could be consolidated, and that through a staged approach, a unified, national, HTA pathway for all health technology evaluation be developed over the medium to long term. This could include a single HTA advisory committee comprising the necessary expertise to assess all applications seeking public funding.

Options to further streamline assessment processes include pathways that calibrate the level of appraisal required for HTA submissions to the level of risk and the clinical need that the submission represents.

### Methods for HTA for Australian Government Subsidy (technical methods)

#### Determination of PICO: pre-assessment processes for selection of comparator(s), and identifying the treatment population and outcomes of interest

The population, intervention, comparator and outcomes (PICO) framework is commonly used in the Australian HTA context to formulate the research question that should be answered by the assessment under four components: population (the population being studied); the intervention(s) (what is being done); the comparators (other main treatment options); and the outcomes (measures of how effective the interventions have been).

While the needs and benefits of defining PICO is generally well accepted by stakeholders, there is some contention across stakeholder groups in relation to definition of elements, in particular the selection of comparators (discussed in the economic evaluation section) and outcomes of relevance to particular populations to ensure equity.

The Reference Committee has therefore proposed options to improve clarity, and stakeholder input into PICO. Refer to the Economic Evaluation section for options relating to the selection of the comparator.

#### Clinical evaluation

The clinical evaluation component of a HTA considers the safety and effectiveness of the proposed health technology in the population of interest relative to the comparator, based on appraisal of the best available clinical evidence. The report commissioned for the Review on [Clinical Evaluation Methods in HTA](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta?language=en) (Paper 4) found that Australia’s approaches were similar to those of other comparable countries and consistent with best practice.

Many stakeholders expressed the view that HTA assessments should allow more flexibility in the evidence base, including greater acceptance of non-randomised evidence and the role of RWD. Many stakeholders felt there was a lack of information about how elements (beyond clinical effectiveness, cost effectiveness and financial impact) such as patient and consumer input were being considered in HTA decision making.

The Reference Committee has observed that a lack of explanation of the clinical evaluation methodologies preferred by decision-makers has contributed to the lack of transparency in clinical evaluation, and proposes options to improve transparency, clarity, and certainty. This includes an explicit, qualitative value framework be developed by the HTA committee in consultation with stakeholders to improve clarity around elements (beyond clinical effectiveness, cost-effectiveness, and financial impact) that the HTA committee considers and how they are considered.

Options have been included relating to updated guidance on the use of non-randomised and observational evidence (including indirect comparisons and the use of ‘Real-World Data’ (RWD) and ‘Real-World Evidence’ (RWE)), other non-traditional evidence, and on the use of surrogate outcomes. We have also proposed options for the development of a curated list of methodologies for clinical evaluation.

#### Approaches for therapies that target biomarkers

We noted that there are a number of challenges relating to biomarkers and genomic profiling under the existing framework for assessing codependent technologies, as well as HTA challenges in relation to the assessment of tumour agnostic cancer therapies resulting in uncertainties, so have proposed options around the development of specific guidance in this area.

#### Economic evaluation

Economic evaluations are a common feature of government priority setting, globally, including in HTA. In Australia, applicants are generally instructed to present either a full cost-effectiveness analysis or cost-utility analysis, or a cost-minimisation analysis, depending on the outcome of the clinical evaluation.

The main concerns expressed by stakeholders about economic evaluation methods relate to how particular elements influence the final estimated value for money of a health technology. Many stakeholders believe the full value of health technologies is not being recognised due to the impacts of elements of economic evaluations such as comparator selection, base-case perspective of evaluation, discount rate, approaches to uncertainty, consideration of societal and equity principles and distributional impacts.

The Reference Committee proposes options to make clearer which comparator should be selected when there are multiple potential comparators and distinguish between submissions claiming superiority and non-inferiority. The committee proposes modelling be developed to determine the aggregate impact of the final HTA Review recommendations that includes different scenarios that vary the discount rate for different types of technologies. It also proposes to consult to understand the broader Australian public views on the circumstances where it may be appropriate to accept higher prices for health technologies.

### Health technology funding, purchasing approaches and managing uncertainty

#### Approaches to funding or purchasing new health technologies and managing uncertainty

Approaches to funding and purchasing health technologies and managing uncertainty were found to vary widely among different countries. However, it was also found that there was nothing appreciably different about approaches used in Australia.

A key issue for stakeholders was how best to manage uncertainty with respect to clinical outcomes, value for money (cost-effectiveness) and the overall financial impact (i.e. cost to patients, the Commonwealth, and state and territory governments) associated with healthcare technologies.

This paper presents options to improve processes for decision making and improve how uncertainty is managed. This includes identification of alternative instruments and financing tools to address budget impact, recognising competition between health technologies that deliver similar outcomes, time-limited funding for therapies that may address areas of high unmet clinical need (HUCN) where there is significant clinical, economic and financial uncertainty, and post-listing reassessment of health technologies.

#### Health technologies that address antimicrobial resistance (AMR)

There is a significant lack of incentive for the development of products that address AMR. Several reasons for this were raised in stakeholder submissions. The Reference Committee presents options to reduce disincentives to seeking funding for antimicrobials and investigates what changes could be made to HTA and funding arrangements to incentivise their marketing.

#### Understanding the performance of health technologies in the Australian setting

The Reference Committee has found that existing approaches for assessing the performance of health technologies require significant further development before they can routinely and reliably be used to assess the performance of health technologies. In particular, systems are inadequate for collecting and integrating the health outcomes data in the real-world setting needed to assess performance.

The Reference Committee believes there are significant opportunities to maximise the value of RWD and RWE for HTA in Australia. It presents a number of options based on the roadmap developed by the MI-CRE in paper 7.

### Futureproofing our systems and processes

#### Proactively addressing areas of unmet clinical need and gaps in funded access

HTA in Australia is reactive. It relies on health technology companies making applications for funding. Australia does not proactively assess unmet clinical need and gaps in funded access or identify whether there are health technologies used elsewhere in the world that could address those needs. Stakeholders supported a more proactive approach to identifying and addressing unmet needs.

The Reference Committee presents a number of options proposed to address these issues, including the development of a priority list of patient indications and horizon scanning to meet that priority list.

#### Horizon scanning

It was identified through interactions with a broad range of stakeholders as well as through the papers commissioned for the HTA Review that having mechanisms for horizon scanning of new therapies prior to their submission would assist with system preparedness, and implementation timelines and result in faster access to patients. However, any program for horizon scanning needs to have a defined purpose for the scanning activity to understand what information is being sought and how it will be used. The committee has proposed a range of options relating to horizon scanning to meet specific informational needs within the health system.

#### Environmental considerations in HTA

Globally, healthcare currently contributes 5% of all greenhouse gas emissions, and decarbonisation is urgently needed. In Australia and other high-income countries, manufacturers of health technology products are making commitments to decarbonise their production processes as part of national efforts to achieve net zero emissions.

In the United Kingdom, the National Institute for Health and Care Excellence (NICE) is working with the British Standards Institution and industry to develop new international standards for carbon footprinting of pharmaceuticals and has committed to developing an approach for considering environmental impacts as part of approval decisions by 2023–24. Canada’s Drug and Health Technology Agency (CADTH) has committed to adapt methodologies and analyses to assess the environmental footprint of technologies as part of its [2022–2025 strategic plan](https://www.cadth.ca/2022-2025-strategic-plan).

The Reference Committee therefore considers that: Australia should actively involve itself in efforts to establish new international standards for assessing the environmental impacts of health technology products, that it consider requiring manufacturers to measure and publicly report emissions of products, and that this information is incorporated into approval and reimbursement decisions in alignment with international best practice and developments in comparable countries.

#### Mechanisms for continuous review and improvement

The Reference Committee notes there has been several updates and other reform processes to HTA since it was introduced in Australia in 1993. Currently there is no scheduled, formal review mechanism for elements and features of the HTA System. To ensure the HTA processes and methods are kept up-to-date and that the system is agile and continues to meet the need of Australians, a process for continuous, rolling review and improvement has been proposed.

#### Capacity and capability in the HTA systems

The Reference Committee notes there are many factors that are creating pressure on the HTA workforce across the healthcare sector. It recognises that work-sharing locally and internationally are important strategies to manage these pressures. This paper presents options to improve capability and capacity of the HTA workforce through internships and strengthen local and international partnerships and work sharing.

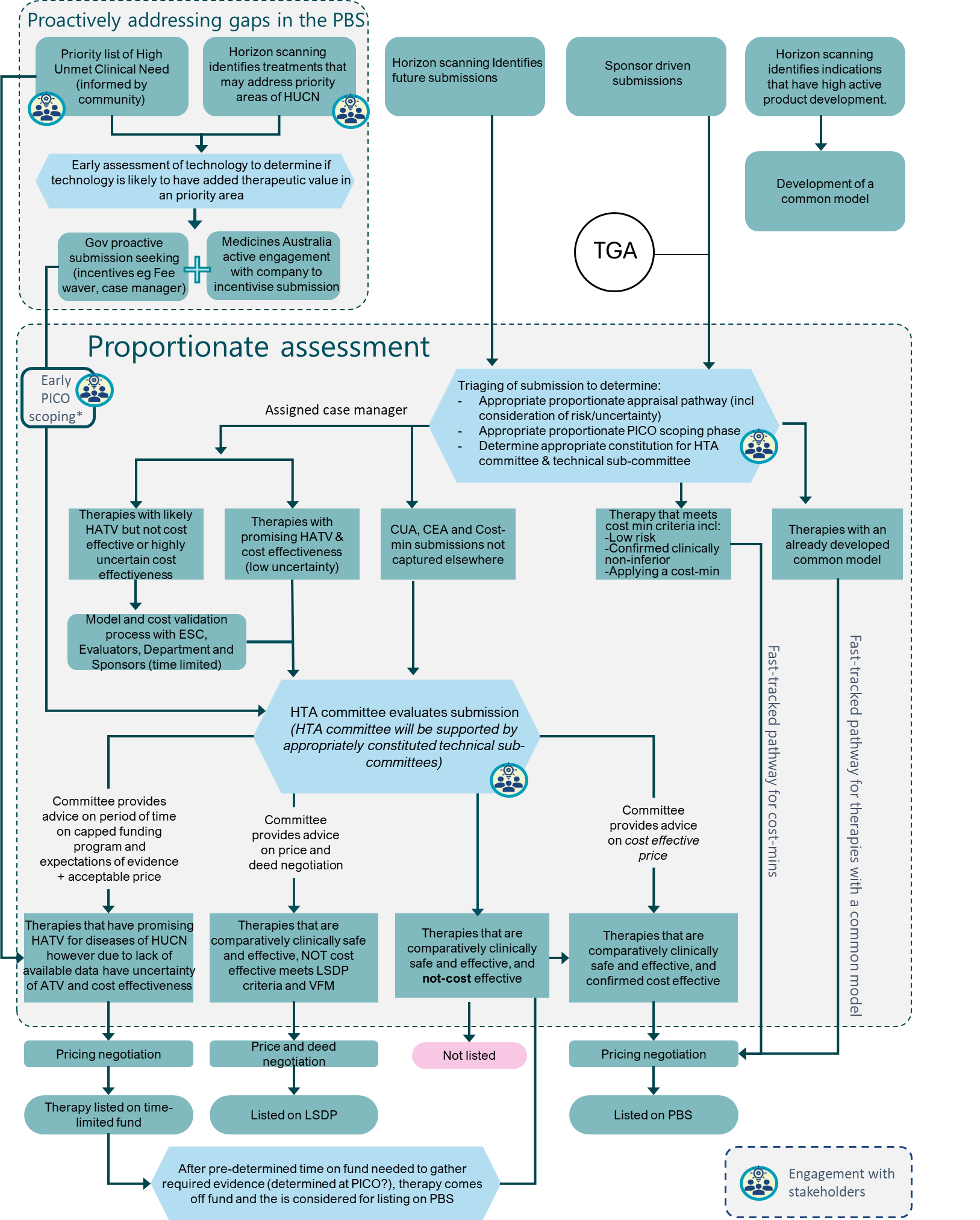
# Overview of the key selected options for consultation

The Reference Committee has comprehensively reviewed the HTA policies and methods in Australia, considering stakeholders' experiences, expert input, and extensive research. The table below sets out options that reflect key proposals made by participants in consultations and the Reference Committee’s consideration of all of the evidence it has received in the HTA Review. The Reference Committee will workshop these options with individuals and organisations with an interest in HTA and any alternative ideas for achieving the same outcomes.

*NOTE: The table contains links to the relevant sections further in the paper. For further information or explanation on concepts, terms or content in the table refer to the main body by clicking the headings.*

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| **Subject** | | | **Key option/s** | |
| 1. Transparency, communication, and stakeholder involvement in HTA | | | | |
| Transparency and communication of HTA pathways, processes and decisions | | | | |
|  | Publish plain language summaries | | 1. 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](https://www.health.gov.au/our-work/co-design-of-an-enhanced-consumer-engagement-process) currently underway) 2. 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. 3. 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 | | 1. 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. 2. Make HTA websites easier to navigate accounting for different levels of knowledge. | |
| Consumer, clinician and other stakeholder engagement and consideration in HTA | | | | |
|  | Develop an engagement framework | | Development of an engagement framework which:   1. 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. 2. 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. 3. 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 | | 1. In addition to a consumer engagement framework, strengthen consumer evidence collection and utilisation by: 2. 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) 3. 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) 4. 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) 5. establishing mechanism or methods to collate patient perspectives formally and routinely 6. including a feedback loop for consumer inputs to show how and where consumers have been consulted and how HTA committees considered this input 7. 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. 8. 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 9. 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 10. 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). | |
| First Nations people involvement and consideration in HTA | | | | |
|  | First Nations peoples partnership in decision making | | 1. Establish a First Nations Advisory Committee to contribute to decision making across the continuum of the below processes: 2. Development of a priority list of population indications with high unmet clinical need (HUCN): 3. 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. 4. 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) 5. Proactive submission request for therapies that are on the priority list (see [Proactively addressing areas of unmet clinical need and gaps in the PBS](#_Proactively_addressing_areas_1)) 6. Include a First Nations representative on the PBAC that can speak to specific benefits for and issues relating to First Nations peoples health 7. 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 | |
| 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](bookmark://_Capacity_and_capability)) | |
|  | 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) | | 1. 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](https://www.health.gov.au/our-work/2020-25-national-health-reform-agreement-nhra). 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 2. 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)   1. Horizon scanning to facilitate timely planning and preparation for adoption by jurisdictions ahead of TGA application being lodged by the sponsor (see [horizon scanning](#_Establishment_of_horizon) below) 2. 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 3. Collaborate with the state and territory governments to ensure results of horizon scanning are being actioned into implementation plans. 4. 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 5. 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. | |
| 1. Health technology funding and assessment pathways | | | | |
| 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. 2. The sponsor of a vaccine makes a submission to the PBAC for the NIP 3. The PBAC evaluators and vaccine evaluation experts evaluate the sponsors submission and produce a single comprehensive assessment report 4. 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 5. 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.*   1. Horizon scanning for vaccines is established including appropriate stakeholders to ensure that ATAGI can be prepared to provide advice. 2. 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 | | 1. 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) 2. 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. | |
| 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 major new therapeutic advances in areas of HUCN | | 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.  Criteria:   1. Therapies that offer likely HATV in areas where there is HUCN, and 2. 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 3. 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))   Alternative option 1: Introducing an optional resolution step **before** HTA committee consideration:   1. 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: 2. progress submission to HTA Committee for consideration ‘as-is’ (with risk of negative recommendation and exit from the HTA cycle); or 3. 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. 4. 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. 5. After the resolution process, the submission would go to PBAC where a recommendation to the minister would be made regarding the listing. 6. This would be a time limited process running contemporaneously to the TGA assessment, resulting in expeditated access. 7. 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. 8. 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).   **OR**  Alternative option 2: Introducing an optional resolution step **before** HTA committee consideration, with additional post committee resolution:   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.   **OR**  Alternative option 3: Early Price negotiation   1. 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   1. 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: 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 the HTA Committee for a second consideration, or 3. agree to ratify the negative recommendation and exit the HTA cycle. 4. With this approach, the relevant advisory committee members, evaluators, Departmental staff and the sponsor would meet and work towards addressing deficiencies / technical concerns. 5. Following the resolution process, the submission would go to the HTA committee where a recommendation to the Minister would be made regarding the listing. 6. This would be a time limited process running contemporaneously with the TGA assessment, resulting in expedited access. 7. 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 high active product development | | 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-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. | |
| 1. Methods for HTA for Australian Government Subsidy (technical methods) | | | | |
| Determination of the Population, intervention, Comparator, Outcome [(comparator is also addressed under economic evaluation)](#_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. | |
| 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](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#page=151) 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](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#page=156) in line with the overarching principles mentioned above.     1. Methods relating to Indirect comparisons: 2. 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. 3. Methods relating to the creation of control groups: 4. Require justification of why an indirect comparison is not possible, or less reliable, than the proposed approach of creating a control group. 5. Require justification for the use of methods that are not prespecified in the study protocol of the proposed technology. 6. Require multiple approaches and/or multiple data sources, if possible, and a discussion of any inconsistencies in estimates. 7. Methods relating to the use of nonrandomised studies - the use of nonrandomised studies to estimate a treatment effect should be: 8. well justified, 9. prospectively designed (preferably in collaboration with HTA or regulatory scientific advice) 10. registered, and 11. supported by multiple sensitivity analyses and transparently reported. 12. Methods relating to adjustment of the treatment effect in the presence of treatment switching 13. 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. 14. 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. 15. Methods relating to the use of RWD and RWE in HTA: 16. 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”. 17. 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. 18. 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: 19. the technology is for use in a population with a HUCN 20. higher quality evidence cannot be generated, or will not be generated in a timely fashion 21. multiple sources of RWE are presented (including both methods of generating RWE from a source, and multiple RWD sources), and 22. 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](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#page=164) 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: 2. Description of the method including links to key peer-reviewed articles 3. 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. 4. 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. 5. Brief explanation for the decision-making committees about how to interpret the results derived by a method. 6. Brief lay explanation of the method for the benefit of patients, clinicians and the broader public. 7. Provide training and guidance to evaluation groups when adopting new methods. 8. 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](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#page=151) 2. Develop a guideline on the assessment and appraisal of genomic technologies and gene therapies for HTA decisions in Australia. 3. 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](#_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). 4. 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. 5. 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 for all health technologies with Commonwealth funding 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. | |
| 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.   Refer: *The operation of section 101(3B) of the National Health Act 1953 – the selection of the comparator*   1. 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. 2. This could include a mechanism to differentiate different type of cost-minimisation submissions based on their proportional benefit. 3. 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 alternitve (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:   1. in what circumstances 2. for what benefit 3. how much greater cost would be reasonable to secure that benefit 4. how confident do we need to be that we will be securing that benefit 5. 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](#_Develop_an_explicit)). | |
| 1. Health technology funding and purchasing approaches and managing uncertainty | | | | |
| 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](#_Options_–_Proportionate)), 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](#_Options_–_Proportionate))](#_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)](#_and_managing_uncertainty) 2. resolving issues in submissions that prevent positive recommendations being made [(see early resolution options)](#_Early_resolution_mechanisms) 3. addressing lack of incentive for developing health technologies in certain areas [(see Approaches to incentivise development of products that address antimicrobial resistance)](#_Approaches_to_incentivise) | |
|  | 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:   1. all health technologies submitted for HTA evaluation; 2. health technologies submitted for HTA evaluation on a cost-minimisation basis; or 3. 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:   * Early identification and nomination via horizon scanning and/or designation on a Priority List of HUCN conditions. * 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) * Requirement for parallel TGA/HTA Committee submission lodgement as part of a broader overall approach to support timely recommendations. * Approach undertaken by the applications and evaluation that: * 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 * 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 uses of different managed entry tools | | 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 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. | |
| 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. | |
| Understanding the performance of health technologies in practice | | | | |
|  | 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. | |
| 1. Futureproofing Australia’s systems and processes | | | | |
| 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](https://www.closingthegap.gov.au/national-agreement) 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 peoples 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*](#po_HS) *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). | |
| 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. (see sState and territory government collaboration in HTA 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: 2. 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 3. understand the potential implications for the Commonwealth from the introduction of these advances in terms of resources, systems and processes. 4. 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). | |
| 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. | |
| Mechanisms for continuous review and improvement | | | | |
|  | A program of continuous review and improvement for current HTA policies and methods | | 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. 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. | |
| 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. | |
| 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:   1. “Work Sharing Initiative” pathway, where concurrent reimbursement submissions are lodged in multiple jurisdictions and dossier modules are work-split amongst participating agencies 2. “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) 3. 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) 4. 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. | |

Figure 1 Schematic highlighting interlinking of selected potential options



# Introduction

## Background to HTAs

Australia’s NMP aims to create the world’s best health, social and economic outcomes for all Australians through a highly supportive medicines policy environment.

This policy is supported by a range of Commonwealth subsidy schemes and funding programs, including the [PBS](https://www.pbs.gov.au/pbs/home) and the [MBS](https://www.mbsonline.gov.au/) – and through the [NHRA](https://www.health.gov.au/our-work/2020-25-national-health-reform-agreement-nhra) between the Commonwealth, state and territory governments.

These initiatives have, for many years, enabled Australians to gain subsidised access to the most effective health technologies to diagnose, monitor, prevent, manage, and treat medical conditions.

Before such health technologies can be marketed in Australia and receive a subsidy, a manufacturer or supplier usually requires marketing authorisation from the Therapeutic Goods Administration (TGA) and funding approval through one of the Commonwealth’s programs or schemes, or via state or territory funding arrangements.

To ensure value for money when deciding whether to subsidise a new health technology, the Government considers advice from independent expert committees comprising medical practitioners, health professionals, health economists and consumer representatives.

When determining their advice, these expert committees consider evaluations that summarise relevant information, including clinical safety, effectiveness and the cost and cost effectiveness of the health technologies compared to alternatives.

HTAs ensure the recommendations provided to the Government (for its consideration) synthesise these elements, enabling decisions on subsidy to be based on the most robust estimates of the health gains produced if a given health technology is purchased at the price offered by the manufacturer or supplier.

## What is HTA?

HTA reviews scientific evidence to assess the quality, safety, efficacy, cost-effectiveness and total costs of health-related goods and services.

These assessments provide policymakers, health funders, health professionals and health consumers with the necessary information to understand the benefits and harms and comparative value of health technologies.

The Government uses HTAs to inform its decisions about which health technologies can be marketed in Australia, and which ones should be subsidised.

Such assessments are commonly undertaken to assess pharmaceuticals (including vaccines, biologicals, and cell and gene therapies), diagnostic tests, medical devices, surgically implanted prostheses, medical procedures and public health interventions.

The 2 main HTA processes are:

* HTA for market authorisation (undertaken by TGA)
* HTA for government subsidy.

TGA assesses the safety, quality and efficacy of new health technologies entering the Australian market and grants market authorisation. TGA authorisation is required before a therapeutic good can be supplied in Australia.

HTAs for government subsidy consider the clinical effectiveness and cost-effectiveness of the new health technology. These HTAs assess the magnitude of the incremental cost benefit and safety of the technology when compared with alternatives, and if this justifies the cost.

Health technologies marketed by the pharmaceutical industry are assessed and subsidised through different pathways and funding arrangements depending on technology type, treatment setting and cost.

## HTAs in Australia

When deciding whether a health technology should be funded or subsidised, the fundamental questions that need to be asked are:

* Does it work?
* Will it work as well as, or better than, what Australians already have access to?
* Will Australians be better off overall if it is funded?

To answer these questions, HTAs consider scientific evidence to assess the quality, safety, efficacy, cost-effectiveness and total costs of health-related goods and services. HTAs allow estimation of the changes in health outcomes arising from the introduction of a new product into the Australian system relative to the costs to the health system if it is funded.

These assessments provide patients and health consumers, policymakers, health funders, health professionals, and health technology companies, with the necessary information to understand the benefits and harms and comparative value of health technologies.

The Australian Government (Government) firstly uses HTAs focused on benefits (efficacy) and harms (safety) to inform its decisions about which health technologies can be marketed in Australia. To then determine which health technologies should be funded, it relies heavily, but not exclusively, on HTAs that focus on the comparative value of health technologies.

Over the past 75 years, the number of health technologies funded in Australia has grown significantly. The first subsidy program, the PBS, was formed in the late 1940s but there are now multiple different Commonwealth, state and territory government programs providing public subsidy for health technologies.

Health technologies have been a major contributor to improved healthy life expectancy over the past century. There has always been a strong case to fund them because the value they deliver for individuals and the community has, for the most part, greatly exceeded the impost on public funds.

Some of the latest technologies have significant promise. They may greatly improve and extend the lives of individuals living with otherwise debilitating and life-shortening conditions. They treat lethal and severely disabling conditions where previously there was no effective treatment available. Some have the potential to do this with a single dose.

They are also becoming increasingly expensive. At the beginning of 2010, the most expensive medicine subsidised through the PBS cost the taxpayer a little over $24,000 each time a patient filled a prescription for the maximum PBS-allowed quantity. In 2015, the most expensive was over $57,000. By 2020, the most expensive was $110,000 whereas, today, the most expensive medicine on the PBS costs a little over $2.5 million upfront.

The decisions to publicly subsidise access to these health technologies are supported by an assessment that: they are likely to work (are safe and effective), likely to work as well as or better than what Australians already have access to (comparatively safe and effective), and the recipient and Australian community as a whole are likely to be better off if it is funded (they represent value for money, and are a better use of taxpayer funds than potential alternative investments).

## Performance of HTA processes in Australia

This section on the performance of HTA processes in Australia should be read in conjunction with [Australian market authorisation, funding and assessment pathways and timelines (Paper 8).](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-australian-market-authorisation-funding-and-assessment-pathways-and-timelines?language=en)

### How Australia compares to other OECD countries

There are several different entities that report on how long it takes for a medicine to be listed in Australia on the PBS. These include:

* the Centre for Innovation and Regulatory Science (CIRS) – HTA Dock[[1]](#footnote-2)
* Medicines Australia – Medicines Matter report[[2]](#footnote-3)
* Pharmaceutical Research and Manufacturers of America (PhRMA) Global Access to New Medicines Report[[3]](#footnote-4)
* Metrics for PBS process improvements.[[4]](#footnote-5)

The CIRS HTA Dock, Medicines Australia Medicines Matter, and PhRMA Global Access to New Medicines reports compare timeframes for subsidy through the PBS with other countries.

The most recent findings of these studies are reported in the paper on Australian market approval, funding and assessment pathways and timeframes. Depending on the timeliness measure, these studies show Australia’s processes rank between the top and the middle of comparable OECD countries.

The methods for analysing and reporting on regulatory and reimbursement approval timelines vary between different entities and studies. This can lead to differences in findings for similar metrics.

Reports on new molecular entities and new active substances authorised and reimbursed are based on the first registered indication in respective jurisdictions. The first registered and funded indication can vary between jurisdictions. Inclusion of repurposed medicines in statistics can significantly skew averages – where for example, the repurposed medicine gained market authorisation many years ago for an indication that was not reimbursed or was later withdrawn and then gained market authorisation for a new indication that was reimbursed. It is unclear to what extent such outliers have been included or excluded in the different reports. The raw data that supports the figures in the CIRS, Medicines Australia and PhRMA reports is not publicly available.

Comparison of the numbers of new medicines reimbursed in different countries can also be misleading as a measure of access as it may not reflect differences between national and local schemes for funding or the availability and performance of alternative health technologies in various countries.

Medicines funded for inpatients in public hospitals in Australia, for example, are unlikely to be captured in international comparisons. In other countries, medicines may be funded but for a smaller population than the approved indication or a subset of the overall population based on level of insurance.

### Actual HTA process timeframes in Australia

#### Timeframes

The timelines for funding a health technology through the PBS, MBS, NHRA, NIP, and under NBA are set out in the paper on Australian market approval, funding and assessment pathways and timeframes. Statistics on actual timeframes for health technologies to be subsidised through the PBS in 2021 and 2022 are also presented in that paper as there has been a sufficient number funded through that pathway to generate meaningful statistics.

There are several decisions and steps that need to be undertaken by different entities between first submission for regulatory approval globally and government subsidy that determine how long it takes for a medicine to be subsidised. The timeframe to a PBS listing from the launch of a new medicine can be broken down into the following broad stages:

1. The time it takes a sponsor to apply for TGA registration after first applying for market authorisation internationally.
2. The time it takes a sponsor to make a submission to the Pharmaceutical Benefits Advisory Committee (PBAC) after it has applied for TGA registration.
3. The time between PBAC submission and the earliest date a pricing offer (PO) can be made after a positive recommendation (this includes the additional time resulting from resubmissions but does not differentiate the number of resubmissions taken)
4. The time it takes a sponsor to make a price offer after the earliest date a price offer can be made.
5. The time it takes for the Government and the sponsor to agree the price and other terms of implementation such as special pricing arrangements, access criteria and sharing of risk.
6. The time it takes for the Government to implement the PBS listing after terms of implementation have been agreed.

Progression between stages requires either a decision from a sponsor, PBAC, the Medical Services Advisory Committee (MSAC) or the Commonwealth Government, or for different entities in the listing process to come to agreement about aspects of the listing.

The time taken for new medicines (new molecular entities) and new indications for existing medicines that listed on the PBS in 2021 and 2022 to progress through each stage varied significantly. These timeframes are set out in [Paper 8](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-australian-market-authorisation-funding-and-assessment-pathways-and-timelines?language=en).

##### Minimum timeframes

The minimum timeframe from PBAC submission to the earliest date for a PO in 2021 and 2022 was 5 months, while the minimum timeframe for all other stages was one month or less. There were:

* examples of sponsors submitting to the TGA before or in the same month as they submitted to the Food and Drug Administration (FDA, US) or European Medicines Agency (EMA)
* examples of sponsors submitting to PBAC in the same month as they submitted to the TGA, and
* examples of new medicines being recommended the first time they were considered by PBAC.

There were no examples of medicines being recommended first time where PBAC ultimately accepted they provided an improvement in efficacy or reduction in toxicity over alternatives. For these medicines, a resubmission or subsequent consideration by PBAC was always required. The minimum timeframe for the listing of a new drug from Australian Register of Therapeutic Goods (ARTG) registration in 2021 and 2022 was 2 months.

##### Median timeframes

In the majority of circumstances, medicines took longer than the minimum possible timeframe to progress through each stage. However, some stages are significantly more variable than others. The stages up to the earliest possible date to make a price offer are highly variable, while the stages after the earliest date a price offer can be made are less variable and range from one to 4 months in the majority of circumstances. The median timeframe for the listing of a new drug from ARTG registration in 2021 and 2022 was 21 months.

##### Maximum timeframes

For each timeframe, there were examples of medicines taking several years to move through stages prior to the earliest date a price offer could be made. After this stage, outliers took several months to move through particular stages. The maximum timeframe for the listing of a new drug from ARTG registration in 2021 and 2022 was 84 months.

#### What are the main contributors to the time it takes to assess and fund a health technology?

The information presented in the paper on Australian market approval, funding and assessment pathways and timeframes reveals that the main contributors to the time it takes to fund a health technology are the following:

* companies seek to market products in Australia only after applying (and/or receiving approval) in the United States (US) and the European Union
* underutilisation of parallel processing
* repeated submissions to advisory committees
* multiple process steps for health technologies funded through the LSDP, NIP and under the NHRA.

##### *Companies seek to market products in Australia after the US and the EU*

For new drugs that were listed on the PBS in 2021 and 2022, the median time between the first EU or US market authorisation application to TGA application was 13 months. While there were some examples of sponsors providing dossiers to the TGA at the same time as the FDA or EMA application, the majority did not.

The Reference Committee heard that Australia pays comparatively low prices for health technologies compared to other countries, which may act as a disincentive in global companies’ decisions to launch new products here. The committee also heard that, compared to the US and the EU, Australia is a very small market and is therefore unlikely to be the first priority for launching new technologies ahead of the EU, US and other large markets, irrespective of our policy settings.

The Reference Committee has not received evidence of specific decisions that would enable it to verify the extent to which these claims are true or what influence they may have on decisions by global companies to market health technologies in Australia.

However, as set out in our options below, the committee thinks it would be worthwhile investigating potential incentives for health technology companies to market products in Australia sooner.

##### Underutilisation of parallel processing

For new drugs and new indications that listed on the PBS in 2021 and 2022, the median time from TGA application to PBAC submission was 9 months. Parallel processing is being used for a majority of submissions but not in the most optimal way. PBAC submissions can be made any time after an application is made to the TGA and there are examples where sponsors have made a PBAC submission in the same month as submitting a TGA application. However, in most circumstances, sponsors do not apply for funding at the earliest opportunity after applying for ARTG registration.

The Reference Committee identified that in some, but not all, circumstances it may be difficult for sponsors to know what the final approved indication will be. The Committee also heard that the requirement that the PBAC receive the TGA Delegate’s overview prior to making a positive recommendation may also contribute to underutilisation of parallel processing.

As set out in our options below, the committee thinks it would be worthwhile investigating potential incentives for health technology companies to apply for funding sooner.

##### Repeated resubmissions to advisory committees before positive recommendation

For new drugs and new indications that were listed on the PBS in 2021 and 2022, the median time from PBAC submission to the earliest possible date a price offer could be submitted following PBAC recommendation was 9 months. Five of these months reflected the time it took to assess the submission and communicate the recommendation, and 1 month to allow the sponsor to submit a price offer following a positive recommendation. The remaining 4 months were the result of submissions not being acceptable the first time they were submitted to the PBAC and consequent requirement for resubmission.

For new drugs where the PBAC ultimately accepted they provided an improvement over alternative therapies, resubmissions added a median of 8 months to the time between PBAC submission and the earliest possible date for a company to submit a price offer. In some circumstances, resubmissions have added years to the time it could take for a medicine to be assessed and recommended by the PBAC.

For new medicines and expanded indications that were considered by the PBAC in 2021 and 2022, the Reference Committee has observed that the main reasons for recommendations not to list were:

* the proposed clinical place in therapy was not accepted
* the proposed clinical place needed to be modified/justified better in a resubmission
* claim of superiority was either not accepted or evidence was not presented or available, and
* fundamental problems in the economic evaluation.

The committee considers that in some circumstances these issues may have been resolved before consideration by the PBAC if there was more time for sponsors to respond and revise their submissions when these issues were raised in commentaries during the evaluation process.

The Committee also observed a correlation between the number of submissions to the PBAC and the size of price reductions in the economic evaluation put forward by sponsors between the first and last submission. The average price reduction between first and last submission for health technologies submitted 2 times was 30%. This increased to 70% between first and last submission for health technologies submitted 4 times. We also note that the unlimited number of submissions and the changes in data, practice, and new entrants into the evaluation processes, may influence and allow the committee’s view to evolve over time on what it eventually considers to be cost-effective.

The Reference Committee agrees with the suggestion from some stakeholders that the main reason for this source of delay to funding new health technologies is that the economic evaluation in submissions is being used as a proxy for negotiation of the prices to be paid for health technologies.

The committee does not think it reasonable to expect this would change for as long as economic evaluations are used both to estimate value and determine prices. But, as is set out in the options below, we do think there is opportunity to resolve issues that prevent submissions being accepted over a shorter period of time. In particular, the committee considers that there are opportunities to enable advisory committees and health technology sponsors to resolve issues and agree on what is a cost-effective price more quickly.

##### Multiple process steps for health technologies funded through the LSDP, NIP and under the NHRA

The processes for funding health technologies through the LSDP, NIP and under the NHRA have more steps than the process for subsidising medicines through the PBS. As is set out in our options below, the Reference Committee sees opportunities to consolidate assessment processes for these technologies to reduce and standardise the time it takes to assess them.

### History of HTA reform processes in Australia

Since HTAs were introduced for the PBS in 1993 there have been several updates and other reform processes to HTA policies and methods. These are listed below:

| **Area** | **Year** | **Reform process** |
| --- | --- | --- |
| **PBAC guidelines updates** | 1995 | Clarification of technical aspects of measuring changes in costs and outcomes |
| 2002 | Update to include minor changes endorsed since November 1995 |
| 2006 | Major revision and reorganisation of text |
| 2007 | Version to provide clarification in subsections A.1, A.5, B.7, C, D.5, F.3, PT1 based on initial feedback on the major revision and to update URLs |
| 2016 | Major revision and reorganisation of text |
| **MSAC** | 2009 | HTA Review |
|  | 2011 | Quality framework |
|  | 2013 | Comprehensive management framework |
|  | 2020 | MSAC Guidelines Review |
| **Other** | 2014 | Post-market review of the LSDP |
| 2018 | LSDP Medicines Review |
| 2017- | PBS Process improvements in the 2017–22 Strategic Agreement between the Commonwealth and Medicines Australia |
| 2020 | House of Representative Inquiry into approval processes for new drugs and novel medical technologies in Australia ([the Inquiry](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report)*)* |
| 2021 | National Medicines Policy (NMP) Review |
| 2022- | HTA process reforms in the 2022–27 Strategic Agreement between the Commonwealth and Medicines Australia |

## Why are we reviewing HTA Policies and Methods?

### Strategic Agreement between the Commonwealth and Medicines Australia

The HTA Review is a commitment in the [2022–2027 Strategic Agreement between the Commonwealth and Medicines Australia](https://www.pbs.gov.au/info/general/medicines-industry-strategic-agreement).

The commitment was made in recognition of the shared goals between the Commonwealth and Medicines Australia to:

* reduce the time for Australians to access new health technologies,
* ensure Australia remains an attractive country for new health technologies to be launched, and
* build on Australia’s status as a world leader in providing access to affordable healthcare.

The aim is to achieve these goals by ensuring Australia’s assessment processes keep pace with rapid advances in health technology and that barriers to access are minimised.

A Reference Committee, which includes an independent Chair, was established to:

* develop terms of reference for the review
* agree to an expert in HTA to undertake an analysis of current methods used by the PBAC, contemporary research and relevant methodologies and purchasing practices used by comparable countries
* oversee public consultations and the analysis conducted by the expert,
* consider submissions to the HTA Review, and
* prepare and deliver a final report and recommendations to the PBAC and the Commonwealth.

### The Standing Committee Inquiry into approval processes for new drugs and novel medical technologies in Australia (the Inquiry)

The Standing Committee Inquiry into approval processes for new drugs and novel medical technologies in Australia ([the Inquiry](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs))*The Inquiry* identified several areas for improvement and set a direction for reform to how Australians access health technologies including HTA.

The Standing Committee did not consider several aspects of HTA policy and methods in depth, noting that they were too technical to be considered properly in the Inquiry. The Standing Committee recommendations included that the HTA Review consider and develop reforms in several areas including:

* for treatments and therapies that do not fit neatly into existing pathways
* cooperation between different HTA and regulatory bodies in Australia and overseas, and with sponsors
* inclusion of patients and clinicians at an early stage in evaluation of submissions
* oversight and reporting on advisory committee decision-making
* use of observational evidence
* selection of comparators, and
* earlier access, including through reduced resubmissions and increased use of Managed Access Programs (MAPs).

The HTA Review will address the issues identified in the Inquiry, and the recommendations of the Standing Committee, where they were agreed in principle by the Government, while also recognising that there are several HTA reform processes that are being undertaken in parallel to the HTA Review.

### Review of NMP

The (NMP) was first published in 2000. During 2021 and 2022, the Department of Health and Aged Care (Department) conducted a review process that included extensive public consultations. A refreshed NMP was delivered to the Australian Government in December 2022 and a [Final Consultation Report](https://www.health.gov.au/resources/publications/national-medicines-policy-final-consultation-report?language=en) is also available. This review recommended new directions for HTAs in Australia to support better healthcare and reduce regulatory costs. It proposes a policy framework for HTA processes for market entry and reimbursement in Australia, including a vision, goal, objectives and principles.

### Patient engagement co-design work – Consumer Evidence and Engagement Unit (CEEU)

A key objective of the CEEU is to expand opportunities for consumers to participate in health technology assessment, thereby enhancing the quality and value of consumer input into HTA processes and decision-making. This work will be informed by the [*Conversations for Change* Report (2023)](https://www.health.gov.au/resources/publications/conversations-for-change-report-2023?language=en), including a focus on improving communication and transparency, and developing guidance and capacity to support consumer engagement in HTAs.

Additionally, the CEEU has responsibility for the [Enhanced Consumer Engagement Process](https://www.health.gov.au/our-work/co-design-of-an-enhanced-consumer-engagement-process), an element of the [2022–2027 Strategic Agreement between the Commonwealth and Medicines Australia](https://www.pbs.gov.au/info/general/medicines-industry-strategic-agreement). This work is being progressed via a consumer-led co-design process informed by consumer, Department and industry representatives and other stakeholders. Recommendations from this process will be provided to the Minister for Health and Aged Care, for consideration.

## HTA Review Overview

### Terms of reference

The [terms of reference for the HTA Review](https://www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-terms-of-reference?language=en) were published on 22 March 2023, taking into account feedback from organisations representing patients, consumers, health technology companies, advisory bodies and state and territory governments.

The terms of reference include objectives relating to what the HTA Review will seek to identify, as well as objectives for the recommendations. including:

*The HTA Review will examine HTA policy and methods, in consultation with stakeholders, to identify features that:*

*1. are working effectively*

*2. may act as current or future barriers to earliest possible access*

*3. may act as current or future barriers to equitable access*

*4. detract from person-centredness*

*5. may be creating perverse incentives.*

*The HTA Review will consider reforms that address identified challenges and present a comprehensive set of recommendations for reforms to Government that:*

*1. are implementable and sustainable for both health funders (Commonwealth, state, and territory) and the health technology industry*

*2. deliver Australians equitable, timely, safe and affordable access to a high-quality and reliable supply of medicines for all Australians*

*3. adopt a person-centred approach in HTA*

*4. deliver the outcomes sought by recommendations from* [*the Inquiry*](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report) *that are agreed in principle in the Government Response*

*5. further the objectives of the new NMP*

*6. ensure HTA policy and methods are well adapted to and capable of assessing new technologies that are emerging or are expected to emerge in the coming years*

*7. do not compromise assessment of patient safety, effectiveness and cost, or advice to Government on subsidy of health technologies. (*[*ToR – page 6)*](https://www.health.gov.au/sites/default/files/2023-03/health-technology-assessment-policy-and-methods-review-terms-of-reference.pdf)

### Public Consultation 1

The first public consultation (Consultation 1) for the HTA Review was open from 11 April 2023 to 16 June 2023.

Consultation 1 received 114 submissions, which included [responses to the online survey](https://ohta-consultations.health.gov.au/ohta/hta-review-consultation1/consultation/published_select_respondent) through the Office of Health Technology Assessment Consultation Hub (Consultation Hub), emailed submissions, and online video forums with the Reference Committee.

#### Insights from Consultation 1:

The University of Technology Sydney’s Centre for Health Economics Research and Evaluation (CHERE) was engaged to produce a [report on the public submissions received for the first round of submissions](https://www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-consultation-1-report).

CHERE noted that all stakeholder sectors indicated support for the rigor applied to the data and processes of HTA, the inclusion of consumer representatives and inputs into that process, and flexibility in decision-making in most cases.

A consistent theme expressed was that there were numerous barriers to people’s timely access to care. These included:

* *that the delays in access to care, particularly the time from registration to funded access for drugs, were too long relative to other comparable countries*
* *delays in (or having no) access to care for those with rare/orphan conditions*
* *a system that is overly focused on randomised controlled trials (RCTs) and the 'economic value', and*
* *a lack of consistent HTA across the Australian healthcare system.*

CHERE reported stakeholders provided a large number of options they believed could improve HTA processes. These included:

* implementing alternative approaches for subsidising technologies (including subsidising some/all technologies at the time of registration, with HTA assessment to follow)
* expanding the scope of evidence considered to give more weight to the inclusion of observational data (RWE)
* incorporating broader aspects of value, in particular patient-reported experience measures (PREMs) and patient-reported outcome measures (PROMs) in assessments of technologies
* facilitating greater community participation HTA
* moving away from a one-size-fits-all approach to HTA to recognise specific technologies and indications, and
* refining the overarching HTA processes to better align them across agencies and allow for non-sponsor-initiated submissions.

### Consultation deep-dives

The Reference Committee held 26 deep dives with individuals from the health technology industry, peak bodies representing consumers, patient advocacy groups, Aboriginal and Torres Strait Islander peoples, clinicians and clinical groups, and state and territory governments.

Deep-dive discussions were aimed at assisting the Reference Committee to gain an in-depth understanding of specific complex topics, issues, challenges, and opportunities for HTA. Expressions of interest for deep dive discussions with the Reference Committee were open to all stakeholders from 16 May 2023 to 1 September 2023.

### Research and Analysis (HTA Expert papers)

Three HTA expert groups were engaged to undertake research and analysis to support the Review. These groups analysed current methods used by Australia’s HTA advisory committees (including the PBAC), contemporary research and relevant methodologies and purchasing practices used by other comparable countries. The organisations and the research they produced are listed below.

Adelaide Health Technology Assessment (AHTA), papers include:

* [Paper 1. International health technology market approval, funding and assessment pathways](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-review-international-health-technology-market-approval-funding-and-assessment-pathways?language=en)
* [Paper 2. Horizon scanning and early value assessment](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-horizon-scanning-and-early-assessment?language=en)
* [Paper 3. HTA Methods: Determination of Population Intervention Comparator Outcome (PICO)](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-determination-of-the-population-intervention-comparator-and-outcome-pico?language=en)
* [Paper 4. HTA Methods: Clinical Evaluation](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta?language=en)

CHERE, papers include:

* [Paper 5. HTA Methods: Economic Evaluation](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-hta-methods-economic-evaluation?language=en)
* [Paper 6. Funding and purchasing decisions and managing uncertainty](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-funding-and-purchasing-decisions-and-managing-uncertainty?language=en)

Centre of Research Excellence in Medicines Intelligence (MI-CRE), papers include:

* [Paper 7. Optimising the availability and use of real-world data and real-world evidence to support health technology assessment in Australia](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-optimised-real-world-evidence-to-support-health-technology-assessment-in-australia?language=en).

### Public Consultation 2 (this consultation)

This consultation seeks to workshop with stakeholders the various options for reform the Reference Committee has heard and developed through Consultation 1, deep dives and in the research and analysis papers.

### Next Steps

Following the second round of consultation (Consultation 2), the Reference Committee will prepare a final report with recommendations. This report will be provided to the PBAC and the Australian Government for consideration.

# Transparency, communication, and stakeholder involvement in HTA

## Transparency and communication of HTA pathways, processes and decisions

### Current state

In Australia, information about HTA pathways, processes and decisions is published on various websites, including those for the PBS (and Medicines Status), MBS, the PBAC, the MSAC, and the Consultation Hub. There is information targeted to patients, clinicians, and sponsors. The different information sources on HTA pathways, processes and decisions are set out in the table below.

| **Web page / target audience** | **Content** |
| --- | --- |
| Medicines Status Website  [www.pbs.gov.au/medicinestatus/home.html](http://www.pbs.gov.au/medicinestatus/home.html)  **Target audience: Consumers / patients** | **Information about the PBS listing process**  High level and detailed information about each listing step  Information about the different decisions that the PBAC may make in relation to submissions  **Submission information**  The status (stage of the PBS listing process) of all submissions considered by the PBAC from July 2019 onwards  Link to public summary of the PBAC’s consideration |
| Consultation Hub  ohta-consultations.health.gov.au  **Target audience: All** | **Items considered by advisory committees**  Information about consultations for items to be considered at PBAC and MSAC meetings including how to make a submission and questionnaires where applicable  Links to agendas for upcoming meetings  **Policy consultations**  Information about consultations for HTA policy matters such as the HTA Review and the Conversations for Change consultations including how to make a submission and questionnaires where applicable  Stores previous consultations and submissions |
| PBS website (HTA pages)  [www.pbs.gov.au](http://www.pbs.gov.au)  **Target audience: All (different documents have different target audiences)** | **Information about making a submission to the PBAC**  Procedure guidance for listing medicines on the PBS and vaccines for the NIP  **Information about PBAC consideration**  PBAC agendas, outcomes, public summaries of PBAC considerations, guidelines for initiating stakeholder meetings, records of stakeholder meetings |
| PBAC website  pbac.pbs.gov.au  **Target audience: sponsors (usually health technology companies)** | PBAC guidelines which provide information on how to prepare a submission, including: proposed use of a medicine, clinical evidence, economic evaluation, extent of use and financial estimates, additional relevant information that may influence decision-making such as equity and access issues and use of expert opinion (from clinicians or consumers) |
| MSAC website  <http://www.msac.gov.au/>  **Target audience: All (different documents have different target audiences)** | **Information about the listing process**  High level and detailed information about each listing step  **Information about how to make an application for MBS funding**  Procedures for making applications and guidelines on preparing applications; MSAC guidelines provide information on how to prepare a submission, including: proposed use of the technology, clinical evidence, economic evaluation, extent of use and financial estimates, additional relevant information such as ethical issues, and consumer evidence and input.  **Information about MSAC consideration**  MSAC agendas, application summaries, public summaries of MSAC consideration, records of stakeholder meetings |

The literature review on [international health technology market approval, funding and assessment pathways (paper 1)](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-review-international-health-technology-market-approval-funding-and-assessment-pathways?language=en) found that decisions were completely transparent for sponsors and partially transparent for patients in Australia, Canada - CADTH, Ireland, Japan, Norway, Singapore, Spain, Switzerland, and the UK.

In Australia, Public Summary Documents commonly contain redactions of clinical and economic evidence.

The literature review found that the Institute for Clinical and Economic Review (also known as ICER) in the US and Belgian Health Care Knowledge Centre in Belgium had complete transparency for both patients and sponsors, but also noted that assessments by these agencies might not directly inform the funding decisions of their payers.

The literature review found that most of the HTA reports relevant to funding decisions are summarised and published with an account of the assessment and the HTA funding recommendations, including in Australia.

### What we heard from stakeholders

Many responses from Consultation 1 expressed negative views about transparency and communication of different aspects of HTA pathways, processes and decisions. The general sentiment was summarised in the [Consultation 1 report](https://www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-consultation-1-report) as follows:

*“An overarching theme affecting many aspects of the performance of the HTA system was transparency with respect to how reimbursement/pricing decisions are made, the factors (evidence) that are considered in making those decisions, and in some cases understanding the overall steps in HTA. While many stakeholders considered that the process for HTA in Australia was generally well described, it was not always clearly understood.*

*For some, the language used in describing HTA processes and requirements was overly technical, obfuscating the nature of that process. For others, there was a lack of information provided on specific aspects of the process (such as how evidence was being combined or weighted in reaching decisions about reimbursement/funding) or without public visibility, impeding engagement with the system and potentially resulting in poorer access.”*

The views expressed in submissions are summarised in the table below:

| **Area** | **Stakeholder views** |
| --- | --- |
| **Pathways** | * *there is not clear information on the steps in the process* * *pathways for new technologies were unclear* |
| **Processes** | * *existing stakeholder consultation and patient engagement mechanisms were insufficient* * *agendas (for PBAC meetings in particular) did not contain sufficient information to help inform what patient organisations should provide* * *guidelines do not adequately describe how certain types of evidence should be presented or will be used in decision-making (such as RWE and RWD; patient, consumer, and clinician input; non-health outcomes; and implementation considerations)* |
| **Methods** | * *it is not clear how non-traditional evidence (e.g. randomised, PROMs) will be accepted as part of the PBAC evaluation* * *the Government could consider providing better bespoke communication to HTA consultancy groups, industry applicants and patients/consumers about the existing provisions in the guidelines for dealing with and presenting a non-traditional evidence base for a technology* * *there is a lack of information or transparency (public visibility) regarding specific aspects of the process (such as how evidence was being combined or weighted in reaching decisions about reimbursement/funding), impeding engagement with the system and potentially resulting in poorer access* |
| **Decisions** | * *public summaries do not communicate information in a way that is accessible and easy to understand* * *public summaries use language that makes them inaccessible for consumers and patients* * *public summaries do not sufficiently communicate what influence certain types of evidence (such as patient and consumer input) had on decisions to recommend or not recommend a health technology b*e *funded* * *it is inappropriate that advisory committees conduct their discussions confidentially* * *it is inappropriate that commercially sensitive information necessary to determine value for money and total cost is redacted from public summary documents* * *it is inappropriate that patients and patient representatives are not included in decision making* * *there are not clear timeframes on the public release of recommendations* * *it is inappropriate that consumers do not have a seat at the table or visibility of negotiations between the sponsor and the Department that determine whether and when a medicine will be reimbursed* |
| **All** | * *communications do not adequately accommodate different levels of health literacy* * *there is inadequate resourcing for consumer tailored and targeted communications and consultations* * *key information is buried in websites* * *difficult to understand performance of the HTA system to inform decision making* |

### Issues

The Reference Committee has observed 3 main contributors to the negative perception of the transparency and communication of HTA pathways, processes, and decisions.

Firstly, the information published about HTA pathways, processes and decisions is extensive, spread over multiple locations, and presented differently in different locations for different audiences and purposes. Stakeholders often cannot find the information they are looking for because it is difficult to find or because it is presented in language, or deals with concepts, which are difficult to understand.

Secondly, there are specific areas where stakeholders would like more or clearer information than is currently published. In particular:

* There are no plain language summaries of PBAC submissions or outcomes as there are for MSAC submissions.
* It is not explicit in advisory committee guidelines and public summaries how evidence that does not form part of the clinical or economic evaluation is factored into decision-making.
* There is limited description of what influence patient and consumer information submitted as part of the consideration of health technologies has on decision-making or what information is most useful to advisory committees.
* Stakeholders are not satisfied with existing plain language explanations of HTA pathways and guidelines.
* There are no timeframes for implementation of health technologies that are jointly funded by Commonwealth, state, and territory governments.
* While stakeholders can see the status of individual submissions through the Medicines Status Website, this information is not presented in a way that enables stakeholders to navigate which submissions are currently under consideration or have been completed to understand how the system is performing.

Thirdly, there are inherent and structural aspects of the HTA that work against transparency such as:

* certain types of information considered by advisory committees (such as that which is commercially sensitive and individuals’ private information) are protected from public release by law
* there is a trade-off between flexibility and clarity (e.g. the absence of a fixed Incremental Cost Effectiveness Ratio (ICER) threshold makes it unclear what would be or would not be acceptable cost-effectiveness)
* the complex nature of the information considered for HTA
* the large volume of information needed to support good decision making – that is, determining whether subsidy of a health technology will benefit Australians and represent a worthwhile use of taxpayer funds
* the large number of applications for funding considered every year
* there are multiple audiences with varying levels of knowledge about HTA, and
* stakeholders are usually not engaged until a submission is made.

### Options to address identified issues

Several improvements could be made to existing communications to help stakeholders get the information they need to improve transparency of HTA pathways, decisions and processes. However, it is acknowledged there will be ongoing challenges communicating HTA pathway processes and decisions due to the complexity of assessing the health outcomes delivered by health interventions, and Australia’s complex arrangements for funding them. We propose reforms later in this paper to simplify HTA pathways that would help consolidate the extensive amount of information currently being published.

The proposed options set out below seek to improve communication of HTA pathways, processes and decisions. Options that may address other arrangements that work against transparency (such as inherent and structural aspects of HTA, and how less readily quantifiable evidence is valued) are considered under later headings.

|  |
| --- |
| **Publish plain language summaries**   1. Summaries of 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. 2. 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. 3. 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). |

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| **Improvements to the HTA webpage including development of a visual, data driven dashboard to increase transparency across the HTA system**   1. 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, TGA and parallel pathway applications, PBAC submission and through to PBS listing. This should be available at the aggregate and individual drug level and be informed by horizon scanning where possible. 2. Make HTA websites easier to navigate accounting for different levels of knowledge. |

## Consumer, clinician and other stakeholder involvement and consideration in HTA

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| **A note on terminology**…  In Australia the term 'consumer', in the context of healthcare, is often used to refer to 'patients, their families/carers, and consumer organisations'. According to the Draft National Consumer Engagement Strategy for Health and Wellbeing 2023: “In the context of health policy, services and care, a consumer is a person who uses, has used or is a potential user of health services and information.  Consumers can participate as individuals, community groups, consumer organisations or consumer representatives.  However, the academic literature refers to the same people as 'patients' or 'patients, carers and patient organisations' reflecting the dominance of the term in international practice (Australia, Singapore and New Zealand being the exceptions).  The literature highlights two different but complementary approaches used in HTA for understanding the needs, preferences, experiences and perspectives of consumers':  1. Research using explicit methods that can be critically assessed (consumer evidence) including Patient Reported Outcome Measures (PROMs) and Patient Reported Experience Measures (PREMs)  2. Participation and input from consumers, ideally 2-way dialogue that is dynamic to enable it to respond to questions as they arise (consumer engagement).  PROMS are those related to the status of a patient’s health condition or treatment that come directly from the patient, examples include health related quality of life, functioning, symptoms (both disease and treatment related), patient satisfaction, and adherence to treatment while PREMs relate to patients’ views and observations on aspects of health care services they have received. |

### Current state

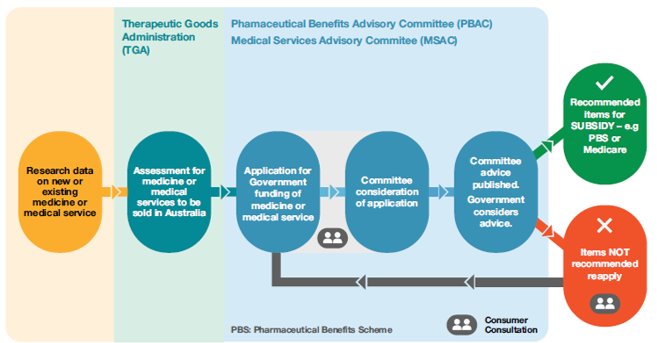
Stakeholder engagement in HTA can include engaging individuals, representatives or groups of patients, carers, clinicians, sponsors and industry broadly, peak organisations, academics and subject matter experts, government bodies, and other members of the public.

For the PBAC and MSAC processes, consumers, health practitioners and any other interested groups or organisations currently have opportunities to provide input either directly or via representation through:

* public consultations on committee agendas
* hearings about specific medicine submissions
* expert clinical consultations (with clinicians and patients who have lived experience of disease) about specific medicine submissions, and
* formal stakeholder meetings on specific health technologies (including post-market opportunities).

Input and evidence from stakeholders are presented by members of the HTA Consumer Consultative Committee (CCC) as part of advisory committee consideration of submissions. As noted in the [Conversations for Change Report](https://www.health.gov.au/resources/publications/conversations-for-change-report-2023?language=en), in recent years, the visibility of patient involvement and consumer representation in HTA has increased. In 2017, the HTA CCC was established, followed by the Department’s CEEU in 2019. These initiatives have improved the ways consumers and patients engage with HTA processes.[[5]](#footnote-6)

Figure 2 Opportunities for consumer consultation in current HTA processes



The HTA Review draft paper on *International health technology market approval, funding and assessment pathways* (Paper 1)[[6]](#footnote-7) compares [stakeholder involvement in the HTA pathways](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-review-international-health-technology-market-approval-funding-and-assessment-pathways.pdf#page=100) across comparable countries including detailed analysis of patient [engagement approaches in HTA](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-review-international-health-technology-market-approval-funding-and-assessment-pathways.pdf#page=107).

The HTA Review draft paper on *HTA Methods: Determination of Population, Intervention, Comparator, and Outcome (PICO)* (Paper 3)[[7]](#footnote-8), [details the approaches for stakeholder involvement in the development of the PICO](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-determination-of-the-population-intervention-comparator-and-outcome-pico.pdf#page=36) in Australia and comparable countries. (Refer to section:Determination of PICO: pre-assessment processes for selection of comparator(s), and identifying the treatment population and outcomes of interestfor further information relating to the PICO).

### What we heard from stakeholders

***Consumers want to be partners. Being ‘equal partners’ with other stakeholders in HTA processes is the main aspirational ‘end goal’ for consumers.[[8]](#footnote-9)***

Throughout Consultation 1, many stakeholders cited the CEEU and CCC as elements of the current system that are working effectively ([Refer to the HTA Review Consultation 1 Report).](https://www.health.gov.au/sites/default/files/2023-11/health-technology-assessment-policy-and-methods-review-consultation-1-report_0.pdf#page=16)

We heard that stakeholders supported systems that allowed consumers, patients and clinicians to make submissions to advisory committees in relation to applications and acknowledged that these were welcomed by advisory committees.

Many stakeholders also felt it was unclear how submissions from consumers are used and what impact they have on the outcome of the application. Stakeholders were concerned that there is no feedback directly to patient organisations about their submission and that submissions from consumers were not published.

We also heard from organisations representing patients and consumers that they found it difficult to prepare submissions due to there being insufficient information regarding the submission available on the agenda (particularly for PBAC submissions).

The stakeholders also felt that engagement with patients and consumers was not occurring early enough, both in the design of clinical trials funded by health technology companies and in the HTA process. Consumer and patient representatives expressed that industry and HTA bodies should be engaging sooner to enable them to help identify priorities and enable greater understanding of lived experience.

### Issues

The involvement of consumers and clinicians earlier and more consistently and formally throughout the HTA pathway would improve the performance and person-centeredness of the HTA system. When stakeholders (e.g. consumers and clinicians) are only engaged from the stage of a HTA submission lodgement it can result in implementation challenges not being identified early which can delay access. Further it can result in the submission missing outcomes of importance to consumers, or in some cases indications for some sub-groups.

The quality of the information provided by consumers, clinicians and other stakeholders to support submissions could be improved if stakeholders were better informed on what information would be most useful to the HTA Committee and how that information could be best presented. Currently, while the information is contained in the public summary documents released after the HTA Committee has made a decision, there is a lack of formalised feedback regarding how stakeholder submissions contributed to the HTA decision in a format that is easily understood by stakeholders broadly. While the CCC does perform this process, it has limited resourcing, and this is not done as a standard course of action.

### Options to address identified issues

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| **Develop an engagement framework**  Development of an engagement framework which:   1. 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, 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. 2. 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. 3. integrates key outcomes of the House of Representatives Standing Committee Inquiry ([the Inquiry](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report)*)* report, [The New Frontier - Delivering better health for all Australians](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report)(The New Frontier report), [Conversations for Change consultation and report](https://www.health.gov.au/resources/publications/conversations-for-change-report-2023?language=en), the [Consumer co-design project](https://www.health.gov.au/our-work/co-design-of-an-enhanced-consumer-engagement-process), and the HTA Review [literature analysis](https://www.health.gov.au/resources/collections/health-technology-assessment-policy-and-methods-review-research-and-analysis-papers) and [consultations](https://www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-consultation-plan). 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 sub-groups 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, and * approaches for managing confidentiality and conflicts of interest. |

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| **Strengthen consumer evidence**   1. In addition to a consumer engagement framework, strengthen consumer evidence by: 2. Adding additional guidance to the [PBAC guidelines](https://pbac.pbs.gov.au/) on the preparation and use of RWD/RWE, consumer evidence (qualitative, PROMs, preferences, PREMs and equity in health (note this is detailed in clinical evaluation recommendations) 3. 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) 4. 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 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) 5. Establishing mechanism or methods to collate patient perspectives formally and routinely 6. Provide a feedback loop for consumer inputs to show how and where consumers have been consulted and how HTA committees considered this input 7. Update 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. 8. 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 9. 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. 10. 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) |

## First Nations peoples involvement and consideration in HTA

### Current state

Under current arrangements, processes for involving First Nations peoples in HTA processes are the same as for other groups impacted by HTA decisions. There is an opportunity for stakeholders to make submissions for funding of new health technologies.

When making a submission, sponsors have the option to include details regarding the impact of the medicine on health equity, including for Aboriginal and/or Torres Strait Islander peoples. However, as the inclusion of this information is at the discretion of the sponsor it is not often included.

Where a decision about funding arrangements is identified as impacting Aboriginal and/or Torres Strait Islander peoples, advice is sought from people (such as Aboriginal and Torres Strait Islander community-controlled health services (ACCHS) representatives) with appropriate knowledge to determine the impact.

To mitigate barriers First Nations people have accessing PBS pharmaceuticals, a range of strategies are used including cost-recovery fee waivers for submissions to the PBAC for medicines for Aboriginal and Torres Strait Islander peoples.

In recognition of their specific health needs, a range of over-the-counter (OTC) medicines are subsidised through the PBS specifically for prescription to Aboriginal and Torres Strait Islander peoples. Special supply arrangements administered under Section 100 of the *National Health Act 1953* allow for PBS medicines to be provided to Indigenous primary health care services in remote locations, at no cost, by a suitably qualified and approved health service professional, without the need for a prescription.

Despite the measures aimed at minimising barriers to access for First Nations peoples, the per-person pharmaceutical expenditure and the PBS-subsidised pharmaceutical expenditure show a disparity is still prevalent. The per-person spend on pharmaceuticals for First Nations peoples was $537 in 2015–16, compared to $891 for non-Indigenous Australians. Spending by the Government through the PBS and the Repatriation Pharmaceutical Benefits Scheme (RPBS) accounted for 31% of total pharmaceutical expenditure for First Nations peoples, and 48% for non-Indigenous people. This represents an average expenditure by the Government of $167 per person for First Nations peoples, compared to $427 per person for non-Indigenous people (2015–16 data)[[9]](#footnote-10).

### What we heard from stakeholders

Stakeholders raised concerns that engagement of ACCHOs and First Nations peoples was *ad hoc* and there was not a dedicated body that fostered these relationships or systematically sought advice on the impact decisions had on Aboriginal or Torres Strait Islander peoples.

Stakeholders consider there are many medicines integral to the health and wellbeing of First Nations peoples that are not listed on the PBS due to a range of reasons including:

* small patient population resulting in low commercial viability meaning sponsors are not making submissions for PBS listing
* sponsors are not making submissions for PBS listing of over-the-counter medications for First Nation peoples because there is no financial incentive
* no mechanism to compel sponsors to identify the needs of subpopulations, and
* complexity with multiple sponsor submissions.

Stakeholders also expressed concern that the absence of a proactive approach to identifying unmet clinical needs and appropriate treatment disproportionately affects First Nations peoples.

Stakeholders also felt there was inadequate support for groups that were not health technology suppliers to bring forward applications for funding and that current arrangements were prohibitive.

### Issues

Lack of formal and routine involvement of a First Nations health representative and consideration of First Nations health outcomes is contributing to health inequity. Advisory committees would benefit from more formal arrangements to ensure the impact of all listing and delisting decisions on Aboriginal and Torres Strait Islander peoples are considered across the HTA system and that a proactive approach is taken to identifying unmet needs and potential therapies that could address them.

This section should be read in conjunction with Horizon Scanning and Early Assessment and International Health Technology Market Approval, Funding and Assessment Pathways

### Options to address identified issues

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| **First Nations people partnership in decision making**   1. Establish a First Nations Advisory Committee to contribute to decision making across the continuum of the below processes: 2. Development of a priority list of population indications with HUCN: In line with the priority reforms under the [National Closing the Gap Agreement](https://www.closingthegap.gov.au/national-agreement) 2020 between all Governments and the Coalition of Peaks, a sub-set of the priority list (see section on Proactively addressing areas of unmet clinical need and gaps in funded access) will be developed in partnership with ACCHSs for the priority areas of HUCN for First Nations peoples 3. Horizon Scanning: An active horizon scanning process 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) (see sections on 4. Proactively addressing areas of unmet clinical need and gaps in funded access and Horizon Scanning) 5. Proactive submission request for therapies that are on the priority list (see section on Proactively addressing areas of unmet clinical need and gaps in funded access) 6. Include a First Nations representative on the PBAC that can speak to specific benefits for and issues relating to First Nations peoples health. 7. Sponsor submissions to require consideration/assessment of the impact on health outcomes for First Nations peoples to enable meaningful informed decision-making. |

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| **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. |

## State and territory government collaboration in HTA

### Introduction and current state

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| ***A note on terminology…***  There are many terms used to describe highly innovative therapies such as (gene modified) cell and gene therapies internationally and in Australia. Internationally, the term Advanced Therapy Medicinal Products (ATMPs) is used to describe medicines based on genes, tissues or cells that treat often very rare and severe disease or conditions in many countries. Here in Australia, the TGA uses the term Advanced Therapies to refer to innovative therapies including cell and gene therapies meeting certain criteria. Some countries and academics use the term Highly Specialised Technologies (including some of the research papers commissioned for the HTA Review) to refer to these therapies. Throughout this document these therapies will be referred to as Advanced Therapies (AT’s).  For the purposes of interpreting the [*2020–25 NHRA Addendum*](https://www.health.gov.au/our-work/2020-25-national-health-reform-agreement-nhra)high cost, Highly Specialised Therapies (HSTs) referred to within that document are:  *“TGA approved medicines and biologicals delivered in public hospitals where the therapy and its conditions of use are recommended by MSAC or PBAC; and the average annual treatment cost at the commencement of funding exceeds $200,000 per patient (including ancillary services) as determined by the MSAC or PBAC with input from the Independent Hospital Pricing Authority; and where the therapy is not otherwise funded through a Commonwealth program or the costs of the therapy would be appropriately funded through a component of an existing pricing classification.”*  While this category in the NHRA Addendum predominately includes advanced therapies, it can also include high-cost pharmaceuticals delivered to public hospital inpatients. However, as per schedule C of the NHRA Addendum, it only deals with funding arrangements for “new high cost, highly specialised therapies (HSTs), that are recommended for delivery in a public hospital setting by the Medical Services Advisory Committee.”  The term HST in throughout this document is generally referring to high cost HST’s delivered to public hospital inpatients and funded under the NHRA. |

The Commonwealth (through the Department) collaborates with state and territory governments through the HTA of high cost, Highly Specialised Therapies (HSTs), which are appraised by MSAC on behalf of both the Commonwealth and state and territory governments. All information in relation to applications for public funding of these therapies that will be jointly funded between Commonwealth, state and territory governments, is shared with state and territory health representatives. State and territory governments are also engaged across all stages of the process including having the opportunity to observe MSAC deliberations and opportunity participate in post-MSAC price negotiations.

Where a high-cost therapy is likely to be delivered in a public hospital, the MSAC and PBAC Chairs, together with a state and territory government representative jointly decide on which committee should assess the application for a new drug or therapy. The MSAC assessment pathway will apply where an application is determined to meet the HST criteria.

For medicines subsidised through the PBS the mechanisms for involving state and territory governments are the same as for other individuals and organisations with an interest in particular submissions.

### What we heard from stakeholders

We heard from state and territory governments that they supported the rigorous processes for assessing effectiveness, safety and cost-effectiveness but that they felt they did not have sufficient visibility of PBAC decisions given the impact on state hospital budgets. Several submissions from state and territory governments expressed they were not appropriately consulted during the process of considering submissions for public funding and making HTA recommendations.

We also heard about the impact of new high cost, or complex to implement therapies and a desire for better planning and clearer timing of implementation.

### Issues

We think there are significant opportunities to increase the participation of state and territory governments in the HTA process. We have heard that state and territory governments (and their hospital systems) collect and hold data on usage and outcomes associated with therapies that are funded or being considered for funding by the Commonwealth that may assist HTA decision-making at the Commonwealth level but is not currently being utilised. There are many reasons for this, including that systems for collecting and analysing data vary across jurisdictions and that there is a lack of centralised data sharing and data standardisation.

We agree that coordinated horizon scanning would help the Commonwealth and state and territory governments plan for disruptive technologies. We also agree there needs to be clarity about implementation, and especially timing, after funding is supported by advisory committees for high cost HST’s, delivered to public hospital inpatients.

### Options to address identified issues

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| **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. |

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| **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](#_Capacity_and_capability)) |

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| **Health Technologies that are jointly funded by the Commonwealth and state and territory governments (such as high cost HST’s delivered to inpatients in public hospitals)**   1. Prioritise and expedite and the development and implementation of a nationally cohesive approach to HTA as outlined in Schedule C of the 2020-25 [NHRA Addendum](https://www.health.gov.au/our-work/2020-25-national-health-reform-agreement-nhra). 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. 2. Establish timeframes for the implementation of HST’s 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)   1. Horizon scanning to facilitate timely planning and preparation for adoption by jurisdictions ahead of TGA application being lodged by the sponsor (see [Horizon scanning](#_Horizon_Scanning) below) 2. 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 HST’s to ensure jurisdictions can begin early implementation planning of HST’s. 3. Collaborate with the state and territory governments to ensure results of horizon scanning is being actioned into implementation plans. 4. 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](#_Proactively_addressing_areas)) 5. 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. |

# Health technology funding and assessment pathways

## HTA pathways and advisory committees

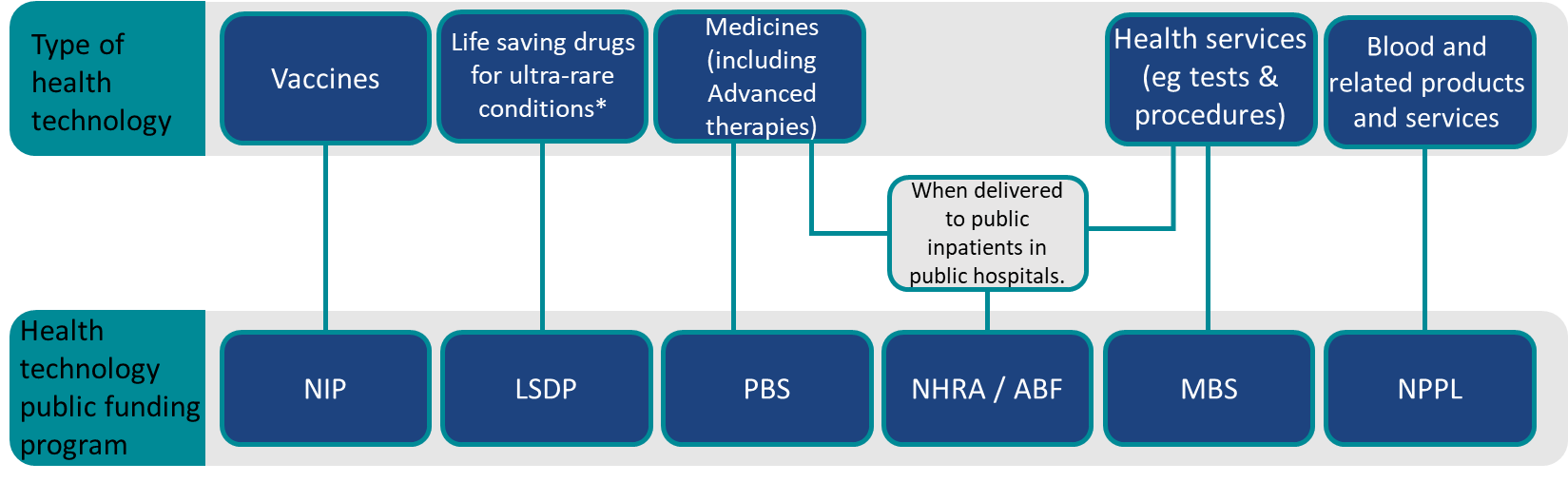
This section should be read in conjunction with [Australian market authorisation, funding and assessment pathways and timelines (Paper 8).](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-australian-market-authorisation-funding-and-assessment-pathways-and-timelines?language=en)

### Current State

Currently in Australia, the funding mechanisms for different health technologies are generally based on the type of health technology and, in some cases, the setting where the health technology will be delivered (e.g. inpatient or outpatient setting).

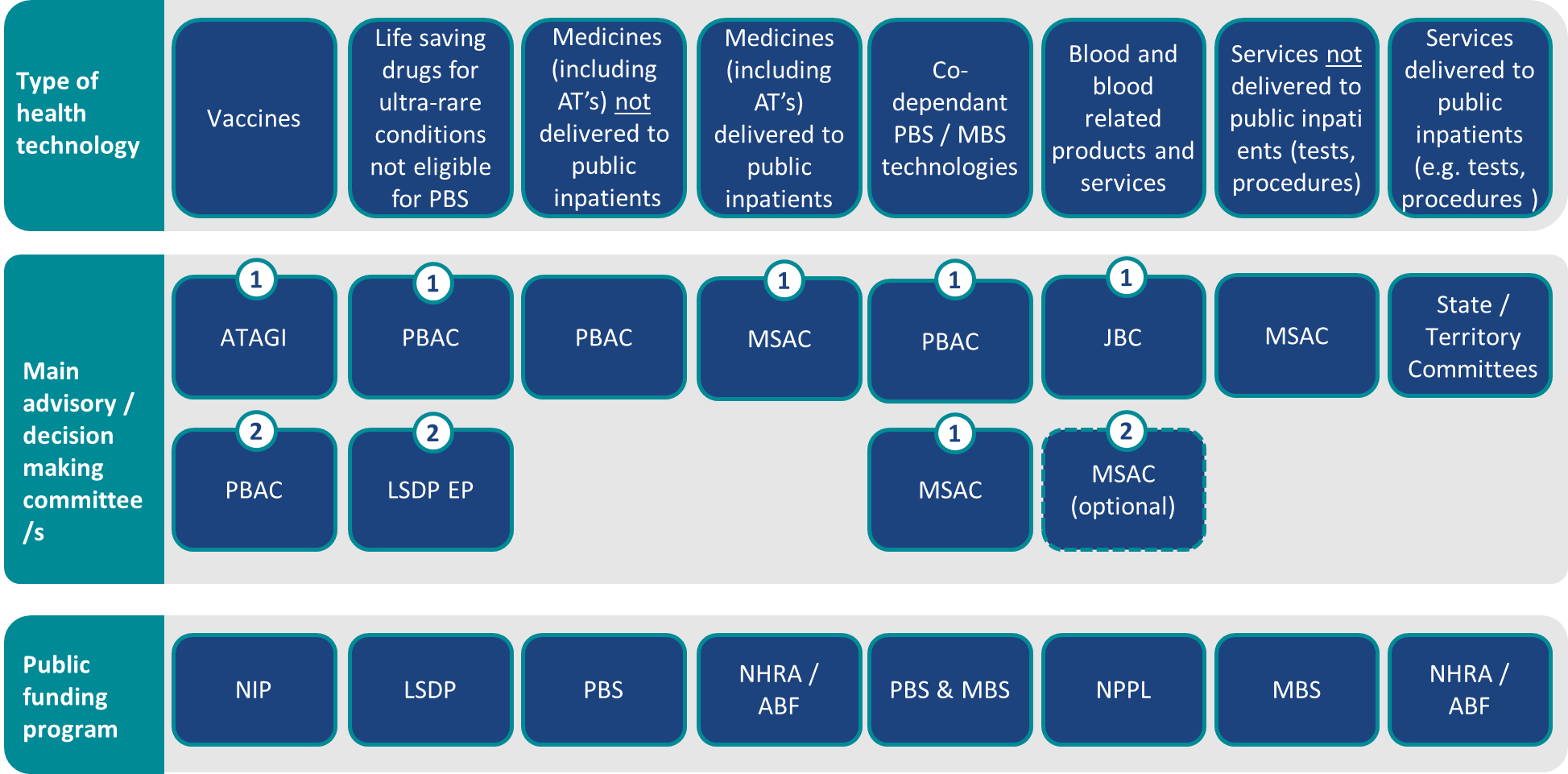
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| ***A note on terminology…***  There are many terms used to describe highly innovative therapies such as (gene modified) cell and gene therapies internationally and in Australia. Internationally, the term Advanced therapy medicinal products (ATMPs) is used to describe medicines based on genes, tissues or cells that treat often very rare and severe disease or conditions in many countries. Here in Australia, the TGA uses the term to refer to innovative therapies including cell and gene therapies meeting certain criteria. Some countries and academics use the term Highly Specialised Technologies (including some of the research papers commissioned for the HTA Review) to refer to these therapies. Throughout this document these therapies will be referred to as *Advanced Therapies (AT’s)*.  For the purposes of interpreting the [*2020–25 NHRA Addendum*](https://www.health.gov.au/our-work/2020-25-national-health-reform-agreement-nhra)high cost, Highly Specialised Therapies (HSTs) referred to within that document are:  *“TGA approved medicines and biologicals delivered in public hospitals where the therapy and its conditions of use are recommended by MSAC or PBAC; and the average annual treatment cost at the commencement of funding exceeds $200,000 per patient (including ancillary services) as determined by the MSAC or PBAC with input from the Independent Hospital Pricing Authority; and where the therapy is not otherwise funded through a Commonwealth program or the costs of the therapy would be appropriately funded through a component of an existing pricing classification.”*  While this category in the NHRA Addendum predominately includes Advanced Therapies, it can also include high-cost pharmaceuticals delivered to public hospital inpatients. However, as per schedule C of the NHRA Addendum, it only deals with funding arrangements for “new high cost, Highly Specialised Therapies (HSTs), that are recommended for delivery in a public hospital setting by the Medical Services Advisory Committee.” |

###### Figure 3 Current state - Health technology main public funding program by type of technology



The funding program for a health technology primarily determines the HTA pathway that will be used to evaluate that technology, including the HTA advisory committee (or advisory committees) that will make recommendations to Government about the funding of that health technology.

###### Figure 4 Current state - Health technology main public funding program and Advisory committee/s by type of technology



These pathways are described in detail in the *paper on approval processes, funding pathways and timeframes.*

### What we heard from stakeholders and Issue Identification

#### General themes

Several key themes emerged from discussions and submissions from stakeholders involved in the current HTA process. While the HTA process is working well in several areas (e.g. the predictability of the 17-week process to initial recommendation, the availability of parallel processing, and the availability of resources to support stakeholders), there are areas where improvement is needed.

Timeliness emerged as a key issue. Streamlining HTA processes to reduce the time from TGA registration to listing on the PBS was emphasised. Stakeholders agreed that using HTA resubmissions as a tool for price negotiation is inefficient.

We heard from stakeholders that timeliness is also a problem for codependent technologies. There is a requirement that each technology in the codependent pairing needs to be considered by the relevant committees (MSAC for the test and PBAC for the medicine) because of the two different funding mechanisms (MBS and PBS). Decisions about the pair of technologies often involve referral between the committees until there is alignment in the funding recommendations.

We also heard that, increasingly, new health technologies are being developed that do not fit neatly into any of our funding pathways. We heard that it was unclear for some technologies which HTA pathway would be used to assess them.

#### Advanced Therapies (including cell and gene therapies)

Cell and gene therapies can currently be assessed for a funding decision either through an MSAC or PBAC HTA pathway. The MSAC and PBAC Chairs, together with a state and territory government representative, jointly decide on which committee should assess the application for a new cell and gene therapy.

The rules for submissions that go through PBAC assessment are set out in the *National Health Act 1953*. As PBS medications cannot generally be dispensed to inpatients in public hospitals (with some exceptions, see [PBS Pharmaceuticals in Hospitals Review](https://www.pbs.gov.au/info/reviews/pbs-pharmaceuticals-in-hospitals-review)) when an Advanced Therapy will be delivered to an inpatient within a public hospital, the assessment of the therapy does not fall within the definition for consideration by PBAC so it is assessed by MSAC. Funding arrangements for high cost Advanced Therapies (also called high cost HST’s in the NHRA) delivered to inpatients in public hospitals are set out in the [2020–25 NHRA Addendum](https://federalfinancialrelations.gov.au/sites/federalfinancialrelations.gov.au/files/2021-07/NHRA_2020-25_Addendum_consolidated.pdf). Under the NHRA, high cost, HST’s that are delivered in to admitted inpatients in public hospitals are jointly funded between the Commonwealth and state and territory governments.

Following a supportive MSAC recommendation, the Commonwealth and sponsor negotiate an overarching Deed of Agreement in line with the MSAC’s conditions for funding of the high cost HST. State and territory governments decide when and where the therapy will be provided.

We heard the following views from stakeholders:

* *A cohesive approach to HTA is particularly important for the equitable access to HSTs in Australia.*
* *Companies seeking to bring new types of technologies such as precision therapies are finding it difficult to determine the appropriate pathway – particularly for gene therapies that could be subsidised either through the PBS or funded under NHRA arrangements.*
* *The assessment landscape is currently fragmented and uncoordinated across assessment entities which can result in applications being assigned to incorrect assessment entities, resulting in increased assessment times.*
* *PBAC decisions often have implications for state hospital budgets, there is currently a lack of transparency in HTA processes, and a risk of jurisdictional stakeholders not being appropriately consulted during the HTA process.*
* *The projected costs for pipeline ATMPs will pose a significant challenge to Commonwealth and state budgets. Unsustainable funding pressures for these therapies would likely be a significant barrier to timely and equitable access, particularly for therapies that are the most unaffordable.*
* *The available evidence supporting these technologies is often limited and insufficient for committee members to determine, with certainty, their comparative safety, effectiveness and cost-effectiveness.*
* *The lack of long-term data in the Australian healthcare context is seemingly placing an increased burden on state and territory health departments in relation to data collection following reimbursement.*
* *Strong post-subsidy technology review/monitoring measures should be developed to manage risks.*
* *Patients and patient organisations note that it is unequitable for access to a therapy to be determined by which jurisdiction you live in (post code lotteries).*

There was a preference from stakeholders to have a single pathway for cell and gene therapies that is both flexible and allows rapid access for patients. There is confusion around having two possible pathways for cell and gene therapies and a lack of clarity about which Committee and pathway would be used.

#### Codependent Technologies

A codependent technology is a medical technology or service that relies on another technology to achieve its intended purpose or enhance its effect. The most common type is a test-medicine pair. To date each technology is assessed by the relevant HTA committee – PBAC assessing the medicine for listing on the PBS and MSAC assessing the test or other technology for listing on the MBS (see [PBAC Guidelines on Codependant technologies](https://pbac.pbs.gov.au/product-type-4-codependent-technologies.html) for more information).

Stakeholders felt that the coordination between the two HTA committees to appraise the paired technologies could be improved as there are sometimes delays occurring as each committee defers to the sister committee for their advice and conclusions before both committees come to a consensus decision.

#### Medicines for ultra-rare conditions

Stakeholders indicated that the existing HTA processes are duplicative for clinically effective treatments for ultra-rare diseases, when they are not recommended by the PBAC based on cost effectiveness and thereby become potentially eligible for the LSDP. The submissions are then considered by the LSDP Expert Panel. This duplication creates unnecessary delays in final decision-making and can delay time-to-access for patients and their carers.

Entry to the existing LSDP pathway requires that a drug is not cost-effective but does not explicitly require consideration of value-for-money. This was believed to complicate price negotiation and reduce transparency in decision-making.

#### Vaccines

The Australian Technical Advisory Group on Immunisation (ATAGI) provide a wide range of advice for government and the public, including contributing to public health recommendations on the use of vaccines. This advice is highly valued and highly respected by patients and patient organisations, industry, clinicians, peak organisations (including National Aboriginal Community Controlled Health Organisation (NACCHO)), and Government.

Across jurisdictions internationally, the evaluation of vaccines frequently involves a different process than that taken for the evaluation of medicines for a funding decision in Australia. Decisions about the use and funding of vaccines often come directly from a National Immunisation Technical Advisory Group (NITAG). In Australia, recommendations for the inclusion of vaccines into the NIP do not come directly from the NITAG (i.e. ATAGI), but also require review by the HTA body (i.e. PBAC). As this process occurs sequentially, this increases the time from TGA market authorisation to an NIP listing.

Additionally, a view held by many stakeholders is that there are duplications between PBAC and ATAGI processes that could be streamlined to remove duplication, reduce administrative burden, increase consistency and improve timely access.

#### New drugs that are non-inferior to alternative therapies

Some stakeholders suggested that new drugs that were non-inferior to alternatives did not require a full HTA and could be assessed over a shorter timeframe. It was also suggested that where these drugs offered no clinically relevant advantage over alternatives that are already funded, they should be funded at a cost that is less than the alternatives as would occur in a competitive market.

### Issues

There is a need to reduce duplication of administration and HTA appraisal processes, increase clarity and certainty, and improve the timeliness of access to health technologies.

The HTA and funding pathways in Australia are more complex than they need to be. The existing separation of pathways based on the funding mechanism is a historical artefact. Further, new health technologies are increasingly challenging the distinctions between different technology types. This has given rise to inefficient administrative workarounds and fragments of expertise between different HTA bodies.

The HTA Committee that assesses a technology should be the one best suited to the technology however currently, it is often the ultimate funding mechanism that determines the HTA Pathway including the advisory Committee. Additionally, the HTA advisory committee not having the flexibility to recommend the appropriate funding mechanism after assessing the technology can result in confusion for sponsors unsure of which pathway to apply through as well as potential miss-alignment that causes work-arounds and further complexity in the system.

While stakeholders suggested creating new, bespoke pathways for some therapies such as cell and gene therapies, the range of potential technology types are too diverse to create further rigid pathways that delineate between different technology types and/or the potentially different needs they may meet. We believe that existing flexibility needs to be maintained to ensure that HTA can adapt to new health technology types that may be marketed in the future.

The current level of appraisal required for submissions is not appropriately calibrated to the amount of risk and benefit that the submission/therapy represents, causing additional strain on limited resources. The level of appraisal should be more flexible to ensure time and resources of Government, evaluators, and sponsors is directed to the submissions where it is most beneficial, and the administrative burden is reduced for low-risk therapies.

Having segmented HTA approaches across the different pathways creates confusion for stakeholders. Having a single-entry point and unifying the approach for all HTA would improve consistency and clarity for stakeholders, however it would require changes to the Department’s HTA processes including a mechanism for triaging submissions to allocate the appropriate HTA Pathway and level of appraisal.

It was identified that some submissions for new drugs claiming non-inferiority do not progress to implementation following a positive HTA committee recommendation due to the price of the comparator being lower than expected by the sponsor. This is largely an issue due to how late in the HTA process sponsors are informed of the price of the comparator chosen in their submission.

Therapies that are likely to deliver major new therapeutic advances in areas of HUCN, often require multiple submissions before they are listed. This is a complex issue with sponsors often relying on the PBAC decision from their first submission as a form of early advice to inform the development of a more fulsome latter submission.

Currently the PBAC is required to wait for the TGA delegate’s overview before communicating its recommendation to the sponsor. This results in an underutilisation of the TGA / PBAC parallel pathway as sponsors will wait to submit their PBAC submission to try to align the PBAC outcome with the expected TGA assessment completion date. This causes delays to access where therapies require a resubmission to the PBAC which could otherwise commence earlier. We also note that while registration of a health technology on the ARTG is necessary for funding new health technologies, the PBAC could potentially provide early preliminary advice.

### Options - Streamlining and aligning HTA pathways and advisory committees

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| Note: This section should be read in conjunction with [Health Technology Market Approval, Funding and Assessment Pathways.](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-review-international-health-technology-market-approval-funding-and-assessment-pathways.pdf#page=10) |

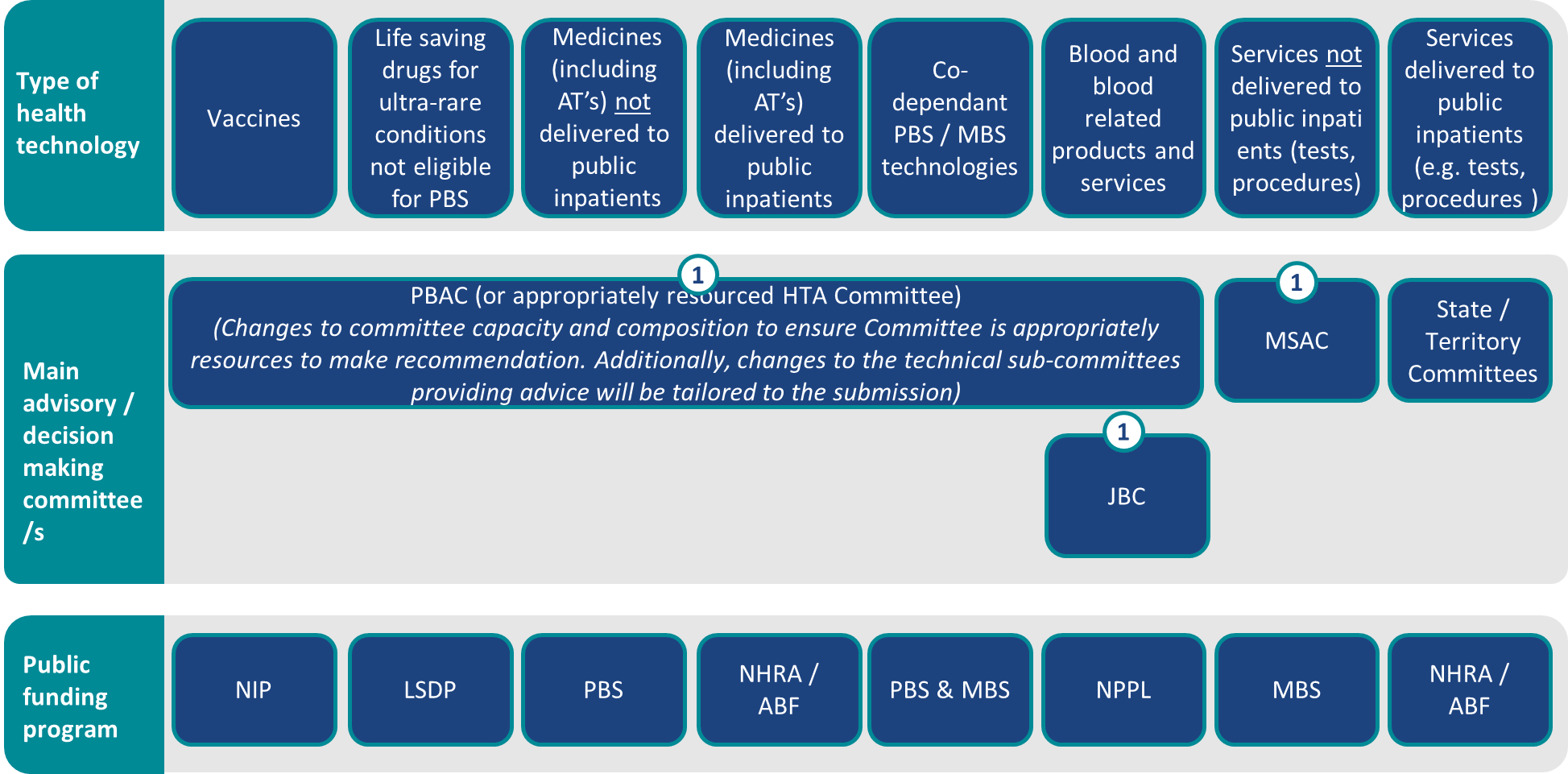
Develop a simplified (single entry) gateway to support efficient lodgement of HTA submissions to support a nationally consistent and cohesive HTA approach; To occur in a staged approach, incorporating the below options:

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| **Pathway for drugs for ultra-rare diseases (LSDP pathway) (short term)**   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.   PBAC advises the Minister on key requirements to enable listing on the LSDP based on a comparative assessment of effectiveness and cost. |

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| **Vaccine pathway (short term)**   1. Streamline the pathway for listing of a vaccine on the NIP by removing the requirement for the sponsor to get ATAGI advice prior to submission. The revised process **would** be as follows. 2. The sponsor of a vaccine makes a submission to the PBAC for the NIP 3. The PBAC evaluators and vaccine evaluation experts evaluate the sponsors submission and produce a single comprehensive assessment report. 4. The ESC is supplemented by the appropriate ATAGI representatives (specialists for particular type of vaccine and disease) to provide formal (ESC + ATAGI) advice to PBAC. 5. 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.*   1. Horizon scanning for vaccines is established including appropriate stakeholders to ensure that ATAGI can be prepared to provide advice. 2. 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.* |

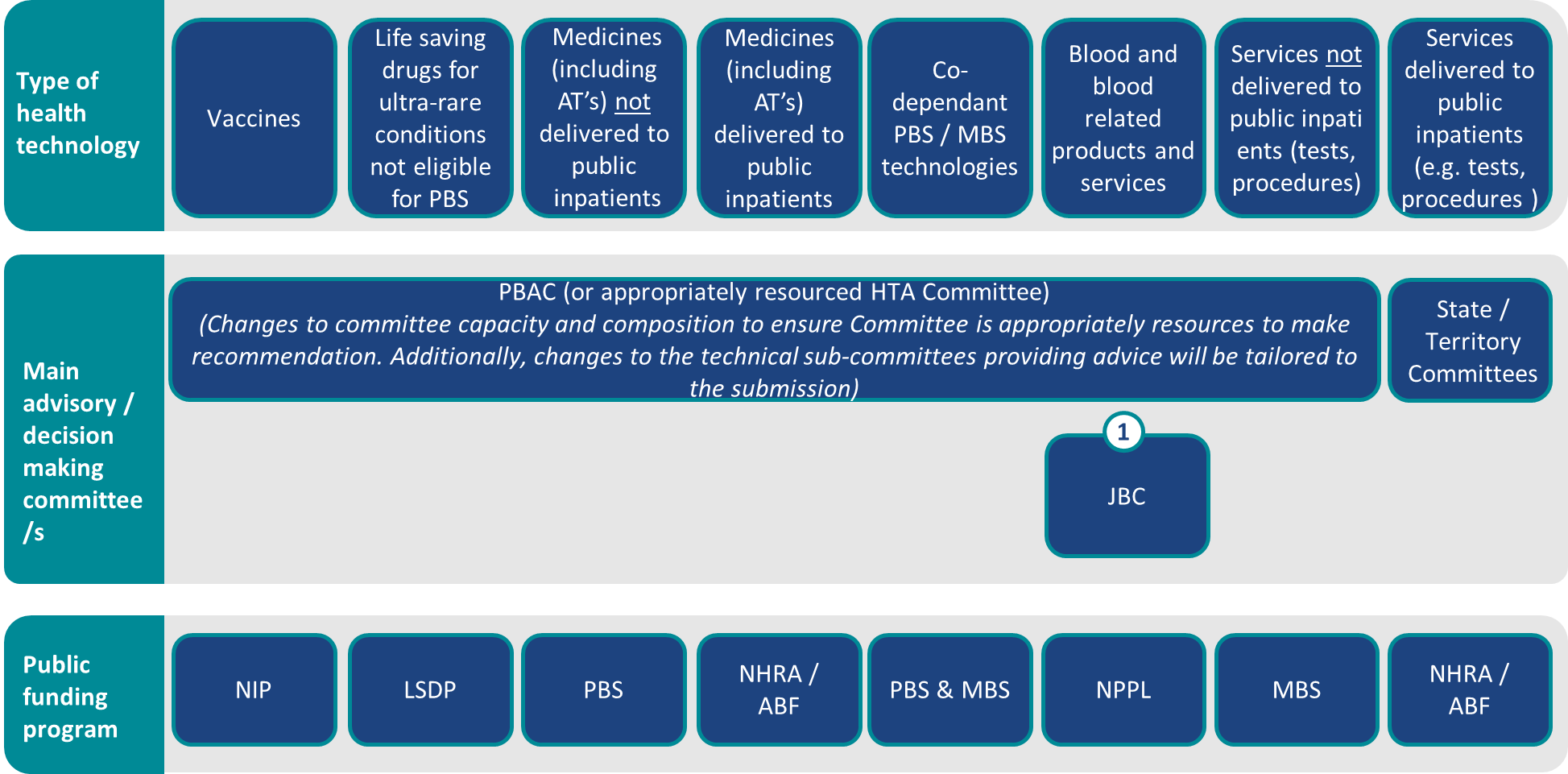
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| **Expanding role of PBAC (medium term)**   1. Further expanding the advisory role of the PBAC to enable it to make the HTA recommendation to the Minister for Health for a broader range of health technologies including codependent health technologies. (short term) 2. The HTA advice does not presume all subsequent funding decisions would take effect through the PBS. |

###### Figure 5 Streamlining and aligning HTA pathway and advisory committee: Short and medium stage changes



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| **Unified HTA pathway for all health technologies with Commonwealth funding (medium to long-term)**  Develop a unified, national, HTA pathway for all health technology evaluation.   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, high cost HST’s delivered to public inpatients), 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 /mechanism 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. |

###### Figure 6 Streamlining and aligning HTA pathway and advisory committee: medium-long stage changes



### Options – Proportionate appraisal pathways

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.

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| **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 before triaging submissions to determine the appropriate evaluation and appraisal mechanisms. Triaging stage would determine:   1. The appropriate appraisal 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), 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. |

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| **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 PBAC Economic Sub-Committee (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 resources. 4. For submissions that do not meet the 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. |

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| **Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN**  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.  Criteria:   1. Therapies that offer likely HATV in areas where there is HUCN, and 2. 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 3. 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))   Alternative option 1: Introducing an optional resolution step **before** HTA committee consideration:   1. 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: 2. progress submission to HTA Committee for consideration ‘as-is’ (with risk of negative recommendation and exit from the HTA cycle); or 3. 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. 4. 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. 5. After the resolution process, the submission would go to PBAC where a recommendation to the minister would be made regarding the listing. 6. This would be a time limited process running contemporaneously to the TGA assessment, resulting in expeditated access. 7. 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. 8. Note: Understanding feasible and practical solutions / strategies to reduce the number of resubmissions under this option 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).   **OR**  Alternative option 2: Introducing an optional resolution step **before** HTA committee consideration, with additional post committee resolution:   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.   **OR**  Alternative option 3: Early Price negotiation  1. 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.   1. 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: 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 the HTA Committee for a second consideration, or 3. agree to ratify the negative recommendation and exit the HTA cycle. 4. With this approach, the relevant advisory committee members, evaluators, Departmental staff and the sponsor would meet and work towards addressing deficiencies / technical concerns. 5. Following the resolution process, the submission would go to the HTA committee where a recommendation to the Minister would be made regarding the listing. 6. This would be a time limited process running contemporaneously with the TGA assessment, resulting in expedited access.   5. 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. |

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| **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. |

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| **Development of a disease specific common model (reference case) for disease areas with high active product development**  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-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. |

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| **Decouple the TGA delegate’s overview from the PBAC meeting**  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 ARTG listing. |

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| **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. |

# Methods for HTA for Australian Government Subsidy (technical methods)

## Determination of PICO: pre-assessment processes for selection of comparator(s), and identifying the treatment population and outcomes of interest

This section should be read in conjunction with [HTA Methods: Determination of the Population, Intervention Comparator, and Outcome (PICO) (Paper 3).](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-determination-of-the-population-intervention-comparator-and-outcome-pico.pdf)

Note: This section relates to the HTA Method of determining the PICO, for additional content on the selection of the comparator see [section on Economic Evaluation](#_Economic_evaluation_1)

### Current state

The starting point for an assessment of a healthcare technology is the formulation of a defined research question that should be answered by the assessment. While there are many different frameworks used in healthcare to define research questions, one of the most common frameworks used in the HTA context is the PICO framework. While the PICO framework had its origins in evidence-based medicine and assisting health practitioners to frame and answer clinical questions it has gained prominence in a range of research settings as a standardised approach to define any given research question.

When used in a HTA context, the PICO framework divides each research question into four components: the population (the population being studied); the interventions (what is being done); the comparators (other main treatment options); and the outcomes (measures of how effective the interventions have been)2.

A properly defined set of research questions (using the PICO framework3) is important because the research question components assist in:

* specifying the evidence that is relevant and eligible to be considered and evaluated in the HTA process
* framing the scope of the HTA evaluation process, and
* guiding the collation, assessment and presentation of the evidence (and resulting answers) that addresses the defined research questions.

In Australia, core elements of the PICO framework are part of the HTA processes as conducted by the PBAC and MSAC. Articulating the PICO components is part of defining the clinical issue and setting the context as to why a health technology should be funded for patient use by the Government.

Articulating the PICO components as part of a HTA submission is generally the responsibility of the applicant in Australia. However, in the MSAC and ATAGI contexts, some PICO criteria development is done by the Commonwealth as part of a “scoping phase”, where input from stakeholders is incorporated to ensure the research question is relevant to the needs of decision-makers and reflects what is important to other stakeholders (particularly patients and clinicians).

### What we heard from stakeholders

A number of stakeholders (principally from the industry sector) observed there is often disagreement between the sponsor and the PBAC on key elements of the PICO, which contributes to the rate of “not recommended” decisions and resubmission churn in the HTA process.

In particular, stakeholders expressed concerns about choice of comparator, and the different pricing-related implications and consequences that feed into the PBAC’s HTA recommendation to the Government (and whether patient access to subsidised health technologies is achieved). This is because different comparators (e.g. lowest cost, best standard of care (SOC), and health technology likely to be displaced in Australian healthcare) can lead to different conclusions in respect of the cost-effectiveness of a health technology in the Australian healthcare context.

Some stakeholders from the research and industry sectors have also noted the lack of a defined PICO confirmation or ratification process for medicine submissions that go through the PBAC process, in contrast to the PICO confirmation step available for submissions that go through the MSAC process and the PICO steps that are part of the ATAGI clinical recommendations process.

Many consumer and clinician organisations raised that the lack of broad stakeholder input into the PICO stage meant that potentially important populations who would benefit from treatment were not identified, as well as a lack of understanding of patient relevant outcomes being considered. While there is a desire for a PICO scoping phase and the inclusion of stakeholder engagement, there was also general acceptance that this should not impact time.

Stakeholders raised that the lack of a mechanism to compel sponsors to identify the health needs of sub-populations (such as First Nations peoples) create is a barrier to equitable access.

PICO criteria (including comparator selection) used to frame HTA submissions remains a point of contention across stakeholder groups, including current legislative interpretation of the *National Health Act 1953* in respect of PBAC’s recommendations to the Government in respect of cost-effectiveness of health technologies for patient populations.

Consumer and clinician organisation raised that the lack of available, accessible (plain language) information regarding the PICO meant that there was often misunderstanding about the intended treatment population and the expected benefit. This resulted in waisted resources providing input, as well as negative health outcomes if patients are waiting for treatments that they may not be eligible for or that may not benefit them. This asymmetric information impacted the ability for consumers to be able to plan for their future in an informed manner.

Industry raised that, for cost-minimisation submissions, they are not made aware of the price of the comparator chosen in their submission until late in the HTA process which often causes resubmissions if it is not economically viable at that price.

### Issues

A lack of timely stakeholder input into the PICO is negatively impacting the performance of the HTA system including increasing the number of resubmissions, important sub-populations not being identified, and a lack of HTA Committee understanding of patient relevant outcomes.

There is a disparity of interests between the sponsor, the Government and patient and clinician communities that impacts the patient population that is included in the PICO, with both Government and sponsors often having an interest in having a narrower patient population. This is problematic when patient populations who would stand to benefit greatly from the additional new treatment are not included in the application for listing. This issue is primary derived from the fact that patient and clinicians are not included in the early discussions about which populations will be included in the submission.

Further, there is a general lack of opportunity for patient perspectives to be incorporated into the PICO, or for participatory dialogue that informs the shaping of PICO criteria to include patient input. There is a lack of transparency relating to the PICO prior to PBAC consideration (such as plain language submission summaries) which impacts patients, clinicians and carers as there is often confusion regarding the intended treatment population, and the benefit of the treatment for different populations/sub-groups.

Divergence on the acceptability and relevance of PICO criteria in the context of HTA evaluation can lead to different recommendations, and may result in increased practical costs, and/or delayed access to certain health technologies for patients.

Currently, the assessment of how new medicines affect health equity is open for sponsors to include at their discretion. Similarly, there is no mechanism to require the consideration of specific priority populations in a HTA submission. This often means that the impacts on health equity of a submission are only included if it is beneficial to the sponsors submission.

### Options

We note the general positive support from stakeholders in respect of the necessity and need for clarity in respect of PICO criteria, as part of supporting high quality HTA submissions and resulting evaluation advice and recommendations to the Government.

We consider reforms that would enable earlier consensus on the PICO to ensure that insights are drawn from a diverse range of stakeholders could improve acceptability of submissions and address concern about stakeholder engagement.

We appreciate that there is particular interest in selection of the comparator for the PICO. This is discussed further in the Economic evaluation section.

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| **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 to be addressed (for new drugs or major expanded indications claiming added therapeutic value). |

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| **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 for patient populations about the proposed treatment population and communicate the expected benefit (outcome) to assist in patients and carers in planning and managing expectations (for new drugs or major expanded indications claiming added therapeutic value). |

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| **Updated guidance**  Updated guidance to require the explicit consideration of 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. |

## Clinical evaluation

This section should be read in conjunction with [HTA Methods: Clinical Evaluation (Paper 4).](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf)

### Current state

The [PBAC guidelines](https://pbac.pbs.gov.au/information/table-of-contents.html) and [MSAC guidelines](http://www.msac.gov.au/internet/msac/publishing.nsf/Content/MSAC-Guidelines) instruct applicants to present the best available clinical evidence to support the effectiveness and safety of the proposed medicine and patient indication. The final output of the clinical evaluation is a conclusion about the effectiveness and safety of the proposed health technology relative to the comparator.

The MSAC Guidelines were substantively revised in May 2021. The PBAC Guidelines were last revised in 2016. The [report on HTA Methods: Clinical Evaluation](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta?language=en) (paper 4) found that Australia’s approaches were similar to those of other comparable countries and consistent with best practice.

The PBAC and MSAC guidelines provide advice on the inclusion of non-randomised studies and non-peer reviewed studies (e.g. clinical study reports). Methods useful for indirect comparisons between RCTs and non-randomised studies are discussed in the PBAC Guidelines.

Both PBAC and MSAC guidelines provide advice on the development of patient pathways including literature sources, the use of an expert panel and the required inclusions in the pathway.

An assessment of long-term safety is also recommended in the guidelines. It is recommended such an assessment includes periodic safety update reports, any pharmacovigilance studies (completed or ongoing post-market surveillance studies), and any studies identified in a separate search, including non-randomised study designs (e.g. registry data and observational studies).

Advice about including consumer evidence (direct patient submissions) is provided in the MSAC Guidelines and described in the [Procedure guidance for listing medicines on the PBS (Version 2.5)](https://www.pbs.gov.au/info/industry/listing/listing-steps). Neither guideline addresses the use of weighting benefits as part of the decision-making process.

#### Best available clinical evidence

##### What is the best available evidence and what evidence can be accepted?

Both guidelines state a preference for clinical and economic evaluations to be based on direct randomised trials, but also provide guidance on the inclusion of non-traditional evidence such as indirect comparisons, non-randomised studies and non-peer reviewed studies (e.g. clinical study reports).

They do not solely require the use of RCT evidence to be provided to demonstrate the clinical benefits of a technology.

Constraints in the available evidence base (such as for health technologies that treat rare diseases and advanced therapies) are recognised and alternative approaches are offered, where required. As observational evidence has traditionally been more likely to be presented to MSAC than PBAC, the [MSAC Guidelines (TG 6.1)](http://www.msac.gov.au/internet/msac/publishing.nsf/Content/E0D4E4EDDE91EAC8CA2586E0007AFC75/$File/MSAC%20Guidelines-complete-16-FINAL(18May21).pdf) provide more extensive information on how to present non-trial evidence, including RWD, and how to assess it.

Both guidelines also express a preference that submissions do not rely on surrogate measures to inform effectiveness in terms of patient-relevant or clinically relevant outcomes. They advise that, where possible, submissions should present evidence from direct RCTs of the treatment effect of the proposed health technology on clinically relevant outcomes.

They also provide guidance for circumstances where direct RCT evidence is not available. This guidance instructs applicants to establish the likely comparative treatment effect on clinically relevant outcomes by transforming the comparative treatment effect of a surrogate measure.

##### Why is data from RCTs considered higher quality than non-traditional evidence?

RCTs are considered the best evidence to demonstrate the efficacy of a health technology as they are designed to address a specific study hypothesis or question and minimise the likelihood of errors that could cause investigators to come to the wrong conclusions about the beneficial and harmful effects of health technologies.

For RCTs, blinding and randomisation controls are used to ensure that bias and confounding is minimised, data are collected purposefully, and data curation is highly regulated.

These controls are especially important in studies where the effects of an intervention are moderate or small. Differences in the characteristics of treated and untreated populations, or a biased assessment of outcomes, has the potential to cause investigators to incorrectly conclude that differences in health outcomes between treated and untreated study participants is due to the intervention.

In contrast, studies using RWD, where data is commonly collected in real-world clinical settings, are subject to bias and confounding and may not completely capture all necessary information. The quality of RWE is multifactorial: it relies on the quality of the underlying data (provenance, reliability and missingness), the quality of the methods used to analyse those data (appropriate study design and analytic methods to control for bias) and the quality of the question itself (data fit for purpose to address the question).

The report on clinical evaluation found that in most jurisdictions, RWE is successfully used to help determine the appropriate comparator, natural history of the disease, treatment pathways, long-term side effects, resource use, incidence, compliance, and quality of life, and for informing some parameters for economic analysis. In most cases, these uses of RWE are well established and accepted.

The report on clinical evaluation found that there are concerns related to the use of RWE to demonstrate treatment effects. Key issues with RWE included:

1. data quality and acceptability
2. bias and confounding
3. lack of training in HTA / methods for evaluation
4. trust and transparency
5. lack of standardisation
6. transferability.

In general, the use of RWE for reimbursement decisions was reported to be limited, driven by a combination of the often-poor quality evidence, and caution applied by payers / decision makers.

#### Consideration of implications beyond clinical effectiveness, safety, and cost-effectiveness

In Australia, the PBAC and MSAC guidelines state that decision-making is informed by less readily quantifiable factors. These include equity, clinical need, severity, value of knowing prognosis or diagnosis, public health issues and other relevant considerations.

The report on clinical evaluation found that broader value elements are considered by most HTA agencies around the world, but that the role and impact of these factors on decision-making lacks transparency.

Several studies identified in the report on clinical evaluation suggested that a method to increase the transparency of the incorporation of broader value elements into decision-making, and improve consistency of decisions, is to use value frameworks such as multiple criteria decision analyses (MCDA).

Three types of value frameworks​ were identified in [Paper 4 HTA Methods: Clinical Evaluation](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta?language=en):

* Qualitative MCDA (qualitative value framework)​
* Quantitative MCDA (each value element for decision-making is scored and then synthesised using pre-determined weights; an overall score is generated)​
* MCDA with decision rules (i.e. value-based decision-rules)​.

[Paper 4 HTA Methods: Clinical Evaluation](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta?language=en) found that systematic incorporation of MCDA *into the HTA methods*, in order to promote equity,appears to be limited globally. Equity is usually considered in a deliberative manner by the appraisal committees​. As with equity, deliberation is a central method for integrating into appraisal decision-making considerations that arise from consumer/patient engagement.​

### What we heard from stakeholders

Many stakeholders expressed a sentiment that HTA assessments should allow more flexibility in the evidence base, and greater acceptance of non-randomised evidence, the role of RWD and surrogate health outcomes.

Some stakeholders felt there could be grater guidance on how to evaluate the clinical and cost-effectiveness of rare diseases where there are evidentiary deficiencies associated with population size and lack of RCT evidence.

Stakeholders also felt there was a lack of information about how elements (beyond clinical effectiveness, cost effectiveness and financial impact) such as patient and consumer input were being combined or weighted in decision-making. Stakeholders perceived this impeded a strong and useful consumer perspective being brought into the decision-making process.

Stakeholders from the research, consultancy and industry sectors expressed that there was a lack of clarity around what information could go into section 5 of the PBAC submission, and how that information should be provided and communicated.

### Issues

The Reference Committee has observed that the evidence base for health technologies and methods for assessing evidence are evolving. We have observed this is being driven in particular by development of health technologies for rare diseases, therapies that target biomarkers and advanced therapies that often have a lower quality evidence base.

We have also heard that methods for assessing non-traditional evidence are becoming more sophisticated and that methods for controlling for potential bias and confounding in these studies are improving.

We consider that these progressive developments necessitate regular review and update of methods guidance.

We have also identified there are a number of areas where applicants would benefit from additional guidance on the preferences of decision makers on the methods used in sponsor submissions.

### Options

These options largely come from the [HTA Paper on HTA Methods: Clinical Evaluation](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#paper=150) (Paper 4). Refer to the paper for further details on the proposed options below.

The adoption of all methods within HTA should follow the following overarching principles. This should be explicit and referred to within the adoption and use of methods for HTA (more detailed explanation available through this [link](https://healthgov.sharepoint.com/sites/HTAReview-External/Shared%20Documents/HTAR%20-%20Options%20consideration/HTA%20Paper%20on%20HTA%20Methods:%20Clinical%20Evaluation)).

1. maintain preference for high quality evidence, where available
2. use fit-for-purpose, transparent methods that are only as complex as required to address the problem
3. justify the use of more complex methods
4. guidance be developed on methodologies preferred by decision-makers
5. evaluation groups be provided training and guidance on new methods
6. feedback be provided to sponsors on their use and presentation of analyses based on more complex methods
7. complex methods that result in estimates that are considerably uncertain may be more acceptable if paired with provisional funding pathways, and
8. acceptability of uncertainty in estimates may be greater in areas of high clinical need.

#### Non-traditional evidence

While we consider a preference for high quality evidence should be maintained, we have also noted the availability of non-traditional evidence such as RWD, RWE, and PROMs is evolving rapidly alongside tools to enable linking and analysis of these datasets (such as artificial intelligence). We consider that guidance on the use of these evidence types as they evolve is needed. In particular, guidance is needed for rare and ultra-rare indications, minor indications, and other disease areas and sub-populations where:

* RCTs may not feasibly be conducted
* RCT comparators do not reflect SOC in local settings
* relevant population groups are excluded from RCTs, and
* major discrepancies exist between RCT and routine practice.

We also consider that guidance on the use of surrogate end points needs to be updated to:

* make clearer the circumstances where surrogates would be acceptable, and
* include methods for describing the uncertainty in the use of surrogate end points.

#### Explanation of preferred clinical evaluation methodologies

We have observed that there is a lack of explanation of the clinical evaluation methodologies preferred by decision-makers, and this has contributed to the perception of a lack of transparency in clinical evaluation. We believe there should be further guidance for methods used in Australian HTAs, outlining the suitability of methods for particular use cases, how to report the method (including supporting data), and how to evaluate the method. We consider that guidance should be regularly reviewed and updated, and therefore it may benefit from being published as a standalone decision support tool outside of the basic information provided in PBAC or MSAC Guidelines.

#### Transparency of decision-making and value frameworks

We agree that more explicit guidance is needed on how elements (beyond clinical effectiveness, cost effectiveness and financial impact) such as patient and consumer input, equity, clinical need, severity, value of knowing prognosis or diagnosis, public health issues and other relevant considerations are being considered by advisory committees and what impact they have on decision-making.

We have observed that other jurisdictions have developed explicit value frameworks for this purpose. Value in this context refers to “consider important”, while the term explicit is intended to mean that the value elements that the committee considers, how they consider them, and what impact the value elements have on decision-making are known.​

​The use of an explicit value framework embeds a patient-centric approach and provides greater confidence that advisory committees are considering factors that are of value to both patients and society. ​​

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| **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](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#page=151) (paper 4) for all HTA Methods. |

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| **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*](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#page=156) (paper 4) in line with the overarching principles mentioned above. These include:     1. Methods relating to Indirect comparisons: 2. 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. 3. Methods relating to the creation of control groups: 4. Require justification of why an indirect comparison is not possible, or less reliable, than the proposed approach of creating a control group. 5. Require justification for the use of methods that are not prespecified in the study protocol of the proposed technology. 6. Require multiple approaches and/or multiple data sources, if possible, and a discussion of any inconsistencies in estimates. 7. Methods relating to the use of nonrandomised studies: The use of nonrandomised studies to estimate a treatment effect should be: 8. well justified, 9. prospectively designed (preferably in collaboration with HTA or regulatory scientific advice) 10. registered, and 11. supported by multiple sensitivity analyses and transparently reported. 12. Methods relating to adjustment of the treatment effect in the presence of treatment switching 13. 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. 14. 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. 15. Methods relating to the use of RWD and RWE in HTA: 16. 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”. 17. 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. 18. 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: 19. the technology is for use in a population with HUCN 20. higher quality evidence cannot be generated, or will not be generated in a timely fashion 21. multiple sources of RWE are presented (including both methods of generating RWE from a source, and multiple RWD sources), and 22. the use of RWE is prespecified in the study protocol for the proposed technology. |

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| **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](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#page=164) 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). |

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| **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: 2. Description of the method including links to key peer-reviewed articles 3. 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. 4. 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. 5. Brief explanation for the decision-making committees about how to interpret the results derived by a method. 6. Brief lay explanation of the method for the benefit of patients, clinicians and the broader public. 7. Provide training and guidance to evaluation groups when adopting new methods. 8. Provide feedback to sponsors on their use and presentation of analyses based on more complex methods. |

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| **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. |

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| **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](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#page=151) 2. Develop a guideline on the assessment and appraisal of genomic technologies and gene therapies for HTA decisions in Australia. 3. 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](#_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). 4. 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.   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. |

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| **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 for all health technologies with Commonwealth funding 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. |

## Approaches for therapies that target biomarkers

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| This section should be read in conjunction with [HTA Methods: Clinical Evaluation (Paper 4), section 7.9.3.](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#page=173) |

### Current state

The indication for most new products relates to a disease or disease subtype. However, it is recognised that many innovative therapies work by targeting biomarkers that are present in multiple diseases and the clinical activity of these products may be more rationally considered for prescription according to whether the biomarker is present or not. Examples include:

* the activity of small molecule inhibitors of cancer-causing genes in multiple rare distinct cancer types, all of which are driven by the same cancer-causing genetic abnormality, and
* the activity of biological agents that can unmask multiple different cancer types so that the immune system can be better activated. These can be variously called tumour agnostic therapies (or pan-tumour or histology independent therapies).

Under current arrangements, health technologies that target biomarkers are assessed as codependent submissions. These submissions seek subsidy for both the health technology and the test for the biomarker. As the use of both the therapy and the test for the molecular biomarker needs to be combined to achieve the intended clinical effect, the net clinical benefits from the joint use of the technologies are assessed. In the clinical evaluation, applicants are asked to provide information about the:

* prognostic effect of the biomarker
* accuracy and performance of the proposed test
* change in clinical management, and
* clinical evaluation of the codependent technologies whether separate or combined.

This approach is partly to assess the validity of the test, and partly to account for the cost of testing to identify a patient with a targetable biomarker.

The PBAC and MSAC guidelines instruct applicants to use different approaches, depending on whether ‘direct evidence’ or ‘linked evidence’ is available.

‘Direct evidence’ describes studies that compare groups of people receiving either the currently used diagnostic test/test strategy or the proposed diagnostic test/test strategy and measures the differential impact of the diagnostic method on patient health outcomes. If patients are randomised to receive the test, then biomarker status would be known and, on that basis, subsequent targeted therapy or usual care could be decided. If patients are randomised to not having the test, then a treatment would be received that is not targeted by the biomarker result.

‘Linked evidence’ describes an approach whereby evidence of test accuracy comparing the proposed and current test/test strategy could be linked (if considered to be appropriately transferable) to separately sourced evidence of treatment effectiveness to approximate the likely clinical effectiveness of the proposed test/test strategy.

For example, this might involve linking evidence of the test’s performance (e.g. diagnostic accuracy) with evidence demonstrating that the test result changes the medicines or treatment prescribed, and with evidence that the alternative medicines have different effectiveness and safety profiles.

### What we heard from stakeholders

Stakeholders expressed a range of concerns about assessment of therapies that target biomarkers. These sentiments included:

* *the need to have them appraised by two committees causes delays*
* *it can be difficult to develop a joint submission on a codependent technology marketed by 2 different sponsors*
* *unclear why the test needs to be evaluated in a codependent pairing, given the often small incremental cost of the test relative to the costs of the targeted treatment*
* *unclear how the codependent technology framework applies to genomic tests*
* *delays in processing gene therapies because of the HST framework*
* *the need to develop evaluation methods and/or different ways of appraising the value of tumour-agnostic therapies.*

### Issues

We note that precision medicine and genomic profiling present a number of challenges to the existing framework for assessing codependent technologies. These include:

* evolution of testing methods, such that a single test may be used for multiple treatments
* as testing becomes more comprehensive, linking the cost of a test to a single medicine may no longer be possible
* that new testing provides more information beyond targeting a treatment to a biomarker, such as providing prognosis or value of knowing information.

We also note there are a number of challenges that have been identified in relation to the assessment of tumour agnostic cancer therapies. These include:

* the use of basket trials (trials involving multiple histologies, commonly with few patients in each basket) and lack of comparative studies
* high uncertainty related to small sample sizes
* the inability to identify and quantify benefits of SOC when historical studies do not present information on the presence or absence of the biomarker
* the inability to perform adjusted indirect comparisons if evidence of a comparator is available
* difficulty in determining the appropriate comparator,
* uncertain estimate of patient numbers (due to uncertain prevalence of biomarkers) which results in uncertain cost of treatment
* uncertain estimate of testing uptake.

[*Paper 4 HTA Methods: Clinical Evaluation*](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta?language=en) found there is currently no preferred HTA approach for evaluating tumour agnostic therapies, and so there is variation in how these technologies are addressed internationally. Approaches in the literature for addressing these problems relate to improving the primary research that is conducted on these therapies, generation of historical or external control arms (which requires data) and making decisions conditional on the generation of RWD to determine treatment performance for conditions that are rare. Of these, only the latter is within the control of a HTA organisation.

We also note that there is sometimes a lack of incentive for health technology companies to seek approval for rarer indications that could plausibly be treated with a health technology already indicated for a more common condition. This lack of incentive arises from poorer evidence demonstrating efficacy treating rarer indications and the small market for such indications.

### Options

We consider that the options we present in this paper to streamline and align HTA pathways and advisory committees, more effectively manage uncertainty though bridging funding, and proactive approaches to inviting submissions to address areas of unmet clinical need would help to address some of the above issues. We would also like to receive feedback on the following options.

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| **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](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-clinical-evaluation-methods-in-hta.pdf#page=151) 2. Develop a guideline on the assessment and appraisal of genomic technologies and gene therapies for HTA decisions in Australia. 3. 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. 4. 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. 5. 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. |

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| **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. |

## Economic evaluation

This section should be read in conjunction with [HTA Methods: Economic evaluation (Paper 5).](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-hta-methods-economic-evaluation?language=en)

### Current state

The PBAC and MSAC guidelines instruct applicants to present an economic evaluation of substituting the proposed therapy with the main comparator in the context of the listing requested.

##### Why do we request economic evaluations?

In Australia, economic evaluations support the Government’s Fiscal Strategy objective of improving living standards for all Australians (Budget 2023–24, Paper 1, 87). They are a tool that is used to assist ministers and government entities to develop their portfolio priorities and manage their budget estimates by helping to ensure quality spending and responsible budget management (Budget Process Operational Rules ([BPORs), Dec 2022, 4](https://www.finance.gov.au/sites/default/files/2023-05/Budget_Process_Operational_Rule_esffective_6%20_December_2022.pdf)). In particular, they help ministers and entities uphold the principle that all expenditure should constitute a proper use and management of public resources, and the most efficient, effective economic and ethical way to achieve the maximum economic benefit for Australians ([BPORs, Dec 2022, 4](https://www.finance.gov.au/sites/default/files/2023-05/Budget_Process_Operational_Rule_esffective_6%20_December_2022.pdf)).

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| The operation of section 101(3B) of the National Health Act 1953 – the selection of the comparator Whenever it is assessing an application for a new medicine, section 101(3B) requires the PBAC to consider:   1. what are the alternative therapies to the new medicine 2. whether the new medicine is substantially more costly than an alternative therapy or therapies, and 3. if so, whether it is significantly more effective, or safer.   Where the lowest cost comparator is an alternative therapy to the new medicine, it cannot be ignored by the PBAC. Doing so could result in recommendation of a medicine that is substantially more costly but not significantly more effective or safer than an alternative therapy, in contravention of section 101(3B).  The PBAC can consider the lowest cost alternative directly or indirectly. An example of an indirect way would be if a new medicine is claimed to be more effective or safer than alternatives on the market, which would include a medicine previously assessed to be more effective or safe than the least cost alternative, the PBAC can use the comparison against that medicine to form a view that the new medicine is also safer and more effective than the least costly alternative.  As described in the second reading speech made when section 101(3B) was introduced in 1987, the then Minister said the provision was included because the Government “did not believe the taxpayer should foot the bill for very expensive drugs that offer only minimal advantages over much cheaper alternatives”. If the PBAC were not required to consider the lowest cost alternative therapy, very expensive drugs could be recommended for listing despite offering only minimal advantages over much cheaper alternatives. |

When providing their advice that supports funding decisions for a health technology that costs more than alternative therapies, advisory committees like the PBAC and MSAC need to be satisfied that it also provides an improvement in efficacy or reduction in toxicity over alternative therapies for some patients. For the PBAC, this requirement is set out at section 101(3B) of the *National Health Act 1953*.

This requirement supports the principle that all expenditure should seek to achieve the maximum economic benefit for Australians by ensuring that where public resources are used to fund a health technology that is more expensive than an alternative therapy, it is because that health technology provides a clinically important advantage over that alternative therapy.

The economic evaluation is the tool that is used to help advisory committees meet this requirement and ensure that any additional cost associated with a health technology is commensurate with the extent of improvement that the health technology provides over alternative therapies.

##### How do economic evaluations in HTA assist decision makers?

Economic evaluations are a common feature of government priority setting, globally, including in HTA. The [literature review on economic evaluation](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-hta-methods-economic-evaluation?language=en) found that economic evaluations are considered by HTA bodies and funding authorities in most other countries to assist decisions about whether to fund health technologies (page 5).

In practice, economic evaluations in HTAs have the following purposes:

* provide an estimate of the value for money of a health technology based on the health outcomes delivered and costs compared to alternative therapies
* help decision-makers understand how confident they should be in those estimates
* are the main mechanism to determine the agreed price paid between the payer and the supplier
* help determine what arrangements should be put in place to manage the risk that estimated benefits are not realised or that costs are greater than estimated.

##### How is the value for money of health technologies estimated in Australia?

There are 2 primary types of economic evaluation that applicants are instructed to present, depending on the outcome of the clinical evaluation.

The PBAC and MSAC guidelines instruct applicants to present a **full cost-effectiveness analysis (CEA)** where the clinical evaluation has concluded that the proposed medicine is:

* therapeutically superior to the main comparator, but likely to result in additional costs to the health system, or
* therapeutically inferior to the main comparator, but likely to result in lower costs to the health system (although this latter circumstance is rarely seen).

The PBAC and MSAC guidelines instruct applicants to present a **cost-minimisation analysis** **(CMA)** where:

* there is a therapeutic claim of noninferiority (or superiority)
* the safety profile is equivalent or superior (in both nature and magnitude)
* use of the proposed medicine is anticipated to result in equivalent or lesser costs to the health system.

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| **Full CEA**  For health technologies that applicants claim are superior (i.e. substantial improvement in efficacy or safety for the technology compared to alternatives), the preferred economic evaluation approaches are CEA and **cost-utility analyses (CUA**) which have similar approaches. The approach taken by Australia is consistent with most countries, where the perspective of the analysis is that of the healthcare funder and there is a reliance on CUA/CEA to estimate the value for money of health technologies.  Main output of the CEA  The main output of a CEA is an estimate of the additional cost for a specified gain in health outcomes (e.g. years of life) from use of the proposed health technology compared to the main comparator. This estimate is referred to as an ICER. In a CUA, the ICER is typically expressed as the cost for each quality adjusted life year (QALY) gained by patients if they use the proposed health technology instead of the comparator.  When preparing a CEA, applicants are instructed to compare the differences in the streams of outcomes and resources that will occur when the proposed medicine or its main comparator are used. These are expressed as the incremental outcomes and incremental costs between these alternatives in the Australian setting.  In some jurisdictions, the ICER determines whether a health technology is funded. In those jurisdictions, if the ICER is below a fixed threshold, the health technology will be funded, and if above, it will not be funded. In Australia, advisory committees do not use a fixed ICER threshold.  Costs and outcomes used to calculate the ICER  The PBAC and MSAC guidelines instruct applicants to include costs related to health and health-related resource use and health-related outcomes. This is often referred to as the “perspective” of the economic evaluation.  The guidelines advise applicants not to include costs and outcomes that are not specifically related to health and/or provision of healthcare in the base case. Such additional costs and outcomes (such as indirect and non-health benefits, and societal implications) can be presented as a supplementary analysis in addition to the base case.  The economic evaluation literature review found that this approach was consistent with approaches used in most other comparable countries. In most countries reviewed, indirect and non-health benefits were not included in the base-case evaluation. The Netherlands, Taiwan and Sweden state the societal perspective is used in economic evaluation for the reference case.  Extrapolation  Trials are generally shorter than patients’ lives with a disease. Consequently, there is a need for extrapolation of the clinical trial evidence, so it may be used to construct a model-based assessment of cost-effectiveness, that reflects the longer anticipated time horizon over which costs and benefits may accrue when the intervention is used in practice. In most countries, including Australia, applicants are instructed to extrapolate by fitting parametric survival models to the observed time-to-event data.  Discounting  The PBAC and MSAC guidelines instruct applicants to discount costs and benefits incurred or received in future years at a uniform compounding rate of 5% per year. Discounting is a method commonly used in economic evaluations to account for society’s preference for costs and benefits now relative to the future. Applicants are also instructed to present sensitivity analyses using fixed discount rates of 3.5% and 0% per year (applied to both costs and outcomes). If relevant, applicants can present supplementary analyses using other discounting methodologies (e.g. a different uniform rate, differential rates, time-varying rates) and justify the alternative approach.  The base case discount rates used in comparable countries ranged from 1.5% to 5% with the majority using less than 5%.  Uncertainty  There are numerous sources of economic uncertainty within HTA, which are generally described to fall in one of 3 broad categories: methodological uncertainty (the normative view about the ‘best’ approach for economic evaluations – including the choice of comparator, discount rate and time horizon), structural uncertainty (the range of assumptions and judgements required in constructing an economic model) and parametric uncertainty (the uncertainty around the mean values of parameters used in the economic model). These sources of uncertainty are compounded where there is clinical uncertainty.  The economic evaluation literature review found that all HTA guidelines address methodological uncertainty through the prescription of either a ‘reference case’ or ‘base case’, which specify the preferred methods in which to undertake economic evaluations. While there are some minor differences between jurisdictions in the extent to which submissions are required to conform to the prescribed reference or base case, all guidelines allow for deviations if they can be justified.  Additionally, all HTA guidelines recommend addressing structural and parametric uncertainty through undertaking some form of scenario or sensitivity analysis. While there is heterogeneity in preferred methods to address parametric uncertainty (deterministic versus probabilistic sensitivity analyses), most guidelines (including Australia (MSAC/PBAC)) provide the option to present both methods.  Weighting of health outcomes and risks/harms  Various methods have been used to assess the trade-offs between health outcomes and risks/harms of interventions. Methods described for weighting health outcomes and risks/harms include MCDA and stated preference methods including conjoint analysis and discrete choice experiments. These methods allow relative weights to be assigned to different health outcomes and healthcare services to reflect their importance for societal impacts and resource allocation.  Australia does not explicitly apply weighting to health outcomes in economic modelling. However, less readily quantifiable factors are considered qualitatively and influence decision-making. The economic evaluation literature review found that HTA bodies in most countries did not explicitly apply weightings to economic modelling and instead considered equity and social factors qualitatively. |

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| **Cost-minimisation analysis (CMA)**  In instances where an applicant claims noninferiority, there is an instruction that the submission includes a cost-minimisation analysis. For this approach, the difference between the proposed medicine and the main comparator is reduced to a cost comparison. The assumption of noninferiority (or superiority) with respect to both effectiveness and safety needs to be well justified for the cost-minimisation approach to be accepted.  Applicants are instructed to provide sufficient detail to establish equi-effective doses and identify any difference between the proposed health technology and the comparator that are likely to result in a difference in health resource use.  The economic evaluation literature review found that several guidelines for HTA recommend cost-minimisation analysis as the main approach to use where no substantial improvement in efficacy or reduction in toxicity is claimed compared to the alternative. |

### What we heard from stakeholders

The main concerns expressed by stakeholders about economic evaluation methods relate to how particular elements influence the final estimated value for money of a health technology.

Stakeholders across industry, patient organisation, research and clinical practice sectors perceived certain features of the economic evaluation methods used by PBAC and MSAC to be causing certain types of health technologies to be undervalued or valued without sufficient regard to societal and equity principles. The elements of the economic evaluation that stakeholders expressed most concern about were:

* low-cost comparators
* non-inclusion of costs and outcomes that are not related to health or provision of healthcare in the base case economic evaluation (e.g. second order effects, social benefits, carer benefits, productivity) and PROMs and PREMs that might not otherwise be captured via standard measures of quality of life
* the impact of discounting and consideration of uncertainty on the estimated value of long-term benefits, and
* absence of an explicit framework incorporating equity considerations into conclusions about whether cost-effectiveness was acceptable.

Stakeholders perceived that undervaluing of health technologies resulted in advice not to fund them at the price initially proposed by the applicant. They expressed concern about the impact this had on the time it takes to reimburse new health technologies.

Stakeholders also expressed concern about the impact of economic evaluation on the time to reimbursement. In particular, some stakeholders expressed the view that it was being treated as a price negotiation step. Other stakeholders expressed concern that for certain health technologies (such as non-inferior health technologies) it was an inefficient use of limited HTA resources.

#### Low-cost comparators

Many stakeholders expressed concern about the impact of low-cost comparators on the estimated value of health technologies. Low-cost comparators can impact the estimated value for money of health technologies regardless of whether the product is non-inferior to existing treatment or is superior to existing alternatives. However, approaches to tackle the issue may be different in these two scenarios and stakeholders addressed these separately in their submissions.

##### Non-inferior health technologies and ‘lowest cost comparator’ policy

Stakeholders expressed concern about the interpretation of section 101(3B) of the *National Health Act 1953* to medicines where the applicant has claimed that it is non-inferior to alternatives. Section 101(3B) gives effect to the requirement that the PBAC only recommends the listing of a drug that is more costly than alternatives if it provides an improvement in efficacy or a reduction in toxicity over those alternatives for some patients. The effect of this interpretation is that where a new drug is claimed to be non-inferior to several alternative drugs, the PBAC will recommend that it is listed on the basis that it costs no more per patient than the cheapest of those alternative drugs. a

An example of this can be found in the PBAC’s consideration of the fixed dose combination of beclomethasone with formoterol for the treatment of chronic obstructive pulmonary disease (COPD) at its March 2022 meeting.

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| **Excerpt: Beclomethasone with formoterol (BEC/FOR) for COPD (PBAC March 2022)**  7.2. The PBAC considered the claim of non-inferior effectiveness and safety to the FDC of fluticasone propionate (FP) 250 µg with salmeterol (SAL) 25 µg was reasonable. However, the PBAC considered for purposes of satisfying Section 101(3B) of the *National Health Act 1953*, any high dose inhaled corticosteroid (ICS) with long-acting beta2-agonist (LABA) FDC are relevant alternative therapies. The PBAC’s recommendation was therefore, among other matters, based on its assessment that the cost of BEC/FOR should be no greater than the lowest price combination of the PBS listed components of ICS/LABA therapy that are available for COPD at comparable doses, irrespective of the days of treatment provided.  7.4 The PBAC considered FP/SAL 250/25 µg FDC to be an appropriate comparator. In addition, the PBAC also considered all ICS/LABA FDCs listed for COPD at comparable doses were appropriate alternative therapies (see paragraph 5.3).  7.10 The PBAC advised that under Section 101(3BA) of the *National Health Act 1953* BEC/FOR should be treated as interchangeable on an individual basis with other appropriate ICS/LABA FDC products on the PBS. |

In its submission to the HTA Review, Medicines Australia stated that this requirement (also referred to as the ‘lowest cost comparator’ policy):

“*a) does not reflect the true value of the new therapy because it does not allow pricing at parity to the most commonly-used alternative, and*

*b) 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.”*

Medicines Australia stated that:

*“this disincentivises innovation and does not reflect the economic value of introducing the new therapy”.*

##### Superior health technologies and low-cost comparators

Several submissions to the HTA Review expressed concern that for indications where there has not been a new treatment brought to market for many years, new medicines are compared to alternative therapies that had been commoditised following patent expiry and were very low cost. Where this occurs, the incremental cost component of the ICER is greater than if there were a newer, higher cost alternative. The effect of this is that health technologies that address longstanding unmet clinical needs may appear less cost effective than health technologies where there has been frequent product development over time and new products have only been compared to patented products.

*“The use of 'current standard of care' [SOC] as a cost comparator for new medicines is a harsh and unrealistic bar to set for diseases in which there is no approved standard of care. In my own area of systemic lupus erythematosus [SLE], no new therapy has been approved/reimbursed in Australia for 60 years, and all currently used drugs are off label and off patent. They are also poorly effective and poorly tolerated, and unmet need for new medicines is acute as young women have a sharply increased mortality. Colleagues in other countries have their patients benefiting now from novel therapies available in their location, while Australian patients are suffering and even dying due to a lack of these. SLE is in the top 10 causes of death in young women. Neither belimumab nor Anifrolumab, two breakthrough drugs now used around the world, have been reimbursed in Australia, and our patients are being left behind. Anifrolumab has been shown to increase attainment of remission and low disease activity, 2 endpoints that are known to be protective from mortality and permanent organ damage. The potential benefit to Australian patients is huge.” (research)*

*“Nowhere is this more evident than in relation to antimicrobials. Novel antimicrobials are generally undervalued by traditional reimbursement systems relative to the benefits they bring to society as indispensable, life-saving drugs. This is because of the existence of low-cost comparators which are still effective for many infections, and the focus of HTA only on direct health costs and benefits.” (industry)*

Anifrolumab for the treatment of severe systemic lupus erythematosus was considered by the PBAC at its March 2023 meeting. The reasons it was not recommended for listing on the PBS are set out below.

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| **Excerpt: Anifrolumab for the treatment of severe systemic lupus erythematosus (SLE) with high disease activity despite standard of care (SOC).**  5.1 The resubmission maintained that SOC alone (placebo) was the main comparator comprising triple therapy with: i) an anti-malarial, ii) an immunosuppressant (minimum dose of methotrexate 20 mg per week, azathioprine 100 mg per day or mycophenolate 1000 mg per day) for at least 12 weeks, and iii) prednisone ≥7.5 mg per day (or equivalent) for at least 4 weeks.  5.2 The PBAC previously considered the nominated comparator was appropriate.  7.1 The PBAC did not recommend the listing of anifrolumab for the treatment of SLE with high disease activity despite SOC. The PBAC acknowledged that there is a clinical need for the requested population but remained concerned that the magnitude of benefit associated with anifrolumab was uncertain. The PBAC also considered that the economic model remained highly uncertain and was unreliable for decision making.  7.5 Consistent with its July consideration, the PBAC considered the claim of superiority to SOC alone in terms of effectiveness was supported based on improvement in disease activity for some patients; however, the magnitude of benefit was modest and uncertain. The PBAC considered the claim of inferior safety to SOC alone was reasonable.  7.7 The PBAC noted that the economic model was informed by an exploratory subgroup analysis of patients in the TULIP trials with a SLEDAI-2K ≥ 10 which reflected the proposed restriction. The PBAC considered this was reasonable; however, noted that there was low confidence in the magnitude of benefit due to heterogeneity of responses between the trials and by outcome, and because of the amendments to outcome assessment that likely favoured anifrolumab.  7.8 The PBAC considered that a revised economic model that addressed the ESC’s concerns, along with a price reduction, would be required to achieve acceptable cost-effectiveness. |

#### Breadth of costs and outcomes included in economic evaluation

Stakeholders expressed concern about the sources of value that could be included in the base case economic evaluation. There was a perception that the focus on the ICER – with the metric of the cost per QALY gained – meant that patient focused values and indirect benefits (such as return to work, return to education and other elements of potential value that might be captured in a social return investment analysis) were not considered or valued in decision making.

*“The HTA system's narrow definition of value and impact poses another barrier to timely access. Traditional measures, such as cost-effectiveness and clinical efficacy, often take precedence, disregarding broader aspects of patient wellbeing and societal impact. By expanding the evaluation criteria to include patient-reported outcomes, quality of life improvements, and the broader socioeconomic benefits of innovative therapies, the HTA system can better reflect the true value and potential impact of these interventions, allowing for more efficient access.” (industry)*

#### Valuing of long-term benefits

Stakeholders were concerned about elements of the economic evaluation that impact the estimated value of benefits that accrue over a long period of time. Stakeholders perceived that the base case discount rate of 5% used by the PBAC and the MSAC and approaches to consideration of uncertainty led to undervaluation of health technologies (such as preventative therapies like vaccines, and gene therapies) that had upfront costs and benefits that accrue over a long period of time.

##### Discount rate

Stakeholders considered the base case discount rate of 5% was too high relative to discount rates used in other countries and was not appropriate for therapies that have upfront costs and benefits that accrue over many years.

*‘If left unchanged, the discount rate 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 life. In Australia, the 5% discount rate has contributed to delays in accessing vital therapies, including vaccines for human papilloma virus (HPV) in adolescents, meningococcal disease in children and adolescents, zoster virus for 60-year-olds and adolescents” (industry)*

##### Treatment of uncertainty

Stakeholders considered that uncertainty about the costs and outcomes was treated too conservatively by advisory committees and government. This particularly impacted treatments that have benefits that accrue over a long period of time where uncertainty about costs and outcomes increases over time.

*“The way in which the PBAC and the Department of Health and Aged Care view and respond to uncertainty and manage risk within HTA evaluations delays access. Multiple submissions may be required because the PBAC aims to eliminate uncertainty through application of the most conservative estimates in both cost-effectiveness and utilisation cost models.” (industry)*

*“One significant barrier to timely access in the Australian HTA system stems from conservative views regarding clinical and economic uncertainty. Decision-making processes often prioritise a cautious approach, particularly when it comes to adopting innovative technologies. In practice this means evaluators will select the most conservative estimate of treatment effect, rather than the most likely. The emphasis on conservative decision-making results in delays, preventing patients from accessing potentially life-changing treatments in a timely manner. When we engage with patients, directly we hear firsthand about the life changing impact of these delays. A shift towards a more balanced approach, considering the potential benefits and accepting a level of uncertainty inherent in emerging therapies, would enable earlier access without compromising patient safety.” (consultancy)*

#### Explicit consideration of factors that may modify estimated value

Some stakeholders expressed concern that there was not an explicit process for assessing equity concerns in PBAC consideration. Some stakeholders considered that economic evaluations should consider the distributional impacts of interventions, and how they affect the health benefits and costs across different subgroups.

*“Issue 1: Insufficient consideration of equity implications in cost-effectiveness and budget impact analysis. These evaluations often fail to explicitly address equity concerns and may not adequately assess the potential differential impacts of interventions on various population subgroups.” (research)*

#### Economic evaluation as price negotiation

Stakeholders raised concern that economic evaluations were being used as a price negotiation where sponsors would put higher prices than they were willing to accept in initial submissions.

*“The cautious approach adopted by the PBAC towards uncertainty can lead to unintended consequences. 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.” (industry)*

### Issues

#### Overall value of health technologies

The Reference Committee has observed that the primary contentions about HTA economic evaluation methods relate to how health technologies are valued. Many stakeholders believe the full value of health technologies is not being recognised due to the impacts of elements of economic evaluations discussed above (comparator selection, base-case perspective of evaluation, discount rate, approaches to uncertainty, consideration of societal and equity principles and distributional impacts). Many stakeholders who made submissions to the review believe that undervaluing of health technologies delays, and in some circumstances prevents, patients from gaining funded access to advances in care through Australia’s funding and subsidy schemes.

We appreciate that where the current SOC is not meeting needs, patients, carers and their treating healthcare professionals would understandably feel frustrated when a potential treatment is not recommended for funding on the basis of unacceptable cost-effectiveness (i.e. value for money), and elements of the value delivered by the health technology appear to have been undervalued or do not appear to have been factored into the decision.

Like many other government investments, health interventions deliver a return to both the individual and society that is greater than the cost of funding them. In other words, the investment delivers a net welfare gain to society. Health interventions have long been considered a worthwhile investment of public funds because they deliver a net welfare gain.

The paper on economic evaluation found that the use of CEAs and CUAs (which estimate value for money by reference to specific health costs and outcomes) by HTA bodies in Australia, and in most jurisdictions overseas, does not involve estimation of indirect and non-health benefits or the overall societal value in the base case. This is a narrower perspective than that used for cost-benefit analyses which are used to value the impacts of other government decisions (e.g.. policy proposals prepared for consideration by the Australian Government).

The narrower perspective used for economic evaluations in HTA in Australia and elsewhere may reflect the fact that economic evaluations in HTA serve dual purposes of estimating value and determining the appropriate price to be paid. This dual purpose has a flow on impact to government priority setting.

For other government investments, the value that an investment delivers to society over its lifetime is not usually the sole determinant of the price. Prices are also determined through competitive market processes or by reference to a price reflecting both the consumer’s and purchaser’s willingness to pay and cost of production. These processes help to ensure that the overall benefit from funding an investment is shared between the public (through net welfare gains) and the producer (profits).

A value-based approach to setting prices is necessary in HTA because HTA bodies and payer agencies do not have access to information that would enable them to calculate cost of production, and even if they did, they would have limited ability to obtain prices by reference to cost of production due to the market power afforded by intellectual property protections for new health technologies.

As the value captured in HTA economic evaluations is the primary determinate of the price agreed between the supplier and the Government, adjusting economic evaluation parameters to increase the recognised value would increase the cost of health technologies and require a greater allocation of public resources to fund them. This would reduce the net welfare gain to society when public resources are used to fund health technologies and increase producer profit.

If the price reflects the maximal value of the health technology to the patient and society, the welfare gain to society from funding the health technology is lost. The relative value for money of funding health technologies would then become less than other potential investments of public resources that would deliver a return to individuals and the community that is greater than the cost of funding them.

Entities responsible for decisions about use of public resources must abide by the principle that all expenditure should constitute a proper use and management of public resources, and the most efficient, effective and ethical way to achieve the maximum economic benefit for Australians (as per Budget Process Operational Rules, or BPORs). Adjusting economic evaluation parameters so that more is paid for health technologies would reduce the net gain and relative economic benefit they deliver compared to other potential government investments that would otherwise be foregone because of the choice to fund health technologies. While there is an opportunity cost resulting from not funding health technologies, or not funding them until an acceptable price is offered, committing to higher ongoing costs for health technologies also increases the opportunity cost arising from displaced investments that would also enhance community welfare.

Over the past decade, successive governments have made a commitment to list on the PBS all new medicines recommended by the PBAC. The listing of medicines on the PBS has received favourable treatment under budget rules that have otherwise required the cost of new expenditure proposals to be fully offset by direct savings to other parts of the budget. This treatment is partly due to successive implementation of statutory price controls that deliver significant savings over the lifecycle of products on the PBS to enable a new medicine’s funding guarantee. This treatment is also supported by the rigorous process for evaluation and price setting used for health technologies which, in turn, ensures a significant (although unquantified) return on investment in terms of welfare gain for society.

#### Relative value of different health technologies

Through Consultation 1 and its face-to-face meetings, the Reference Committee heard evidence that certain features of the economic evaluation methods make certain types of health technologies appear less cost effective than other types of health technologies. The main categories identified were:

* new health technologies where the comparator is either an old SOC or a health technology that has been commoditised (and is therefore cheap)
* preventative treatments (such as vaccines and gene therapies) that have high upfront costs and benefits that accrue over a long period of time.

We have also heard from a number of stakeholders that there should be an explicit approach to modifying thresholds and/or decision-making based upon equity principles.

##### Cheap comparator (health technologies that are superior to alternatives

For health technologies claimed to be superior to alternatives, the incremental cost per QALY gained is much greater when the comparator is cheap than when it is expensive. This means that the ICER for a new health technology compared with a cheap comparator will appear to be less acceptable than an ICER for a new health technology compared with an expensive comparator – even where the price and the number of QALYs gained are equal for both health technologies.

For medicines where it has been demonstrated they offer an improvement over the old SOC or cheap comparators, the flexibility afforded by the absence of an ICER threshold and interpretation of economic models enables advisory committees to account for this issue when satisfying themselves that a health technology is cost-effective. This is illustrated by the fact that some of the highest priced medicines on the PBS (such as nusinersen for spinal muscular atrophy which has a PBS dispensed price of $110,000 per injection) were assessed against placebo or standard of care.

There is no explicit guidance on how this flexibility is exercised in PBAC or MSAC guidelines. This contributes to a perception that health technologies compared to a cheap SOC are less likely to have acceptable cost effectiveness than health technologies compared to an expensive SOC.

Absence of explicit guidance on how this situation is managed may create a perverse incentive for investment in development of health technologies where comparators are high cost as opposed to those where comparators are low cost. While policy settings in Australia would not necessarily influence decision-making by health technology companies at a global level, this issue is not unique to HTA in Australia and impacts all HTA settings where CEA and CUAs are used.

This issue may be contributing to the relatively high investment by health technology companies in developing treatments for cancer (where there has been continual development of new treatments over time and therefore higher cost comparators) over other areas such as AMR and rare diseases (where there has been little development and the comparators are old and relatively low cost). Although, it should be noted that the relatively small markets for new antimicrobials and treatments for rare diseases would also contribute significantly to the lack of incentive to develop new products in these areas.

##### Cheap comparator (health technologies that are non-inferior to alternatives

Where a health technology is non-inferior to (no more safe or efficacious) than alternative therapies, funding can only be recommended on the basis that it will cost no more than the cheapest of those alternative therapies.

This does not mean that the health technology must be funded at the cost of an inferior therapy for the condition. If an advisory committee such as the PBAC is satisfied that a health technology is non-inferior to alternative therapies that are better than an inferior therapy, it can cost more than the inferior therapy, provided it does not cost more than the cheapest of the alternative therapies that it is not better than.

This requirement becomes controversial where the supplier of the health technology has an expectation that it will be funded at a higher price than the lowest priced alternative therapy that is not the most likely to be replaced in clinical practice and this expectation is not met. In these circumstances, suppliers may choose not to proceed with the application for funding. This can occur where:

* the supplier has claimed the health technology is superior to alternative therapies and the advisory committee is not satisfied the claim of superiority is supported by the clinical evidence presented in the submission
* the comparator is subject to a special pricing arrangement and its price is significantly lower than the supplier expects, or
* the advisory committee considers a cheaper comparator that is not the comparator chosen by the supplier is a relevant comparator (on the basis of a belief that it is also non-inferior to the health technology in the submission).

Where there is minimal difference in health outcomes delivered by the health technology compared with alternatives that are already funded, there is little impact on access to appropriate treatment for patients. However, there may be clinical areas where patient response to treatment is heterogeneous and for clinical reasons having a range of treatment options (that might all deliver the same health outcomes at a population level but have different impacts on individual patients) is necessary for achieving overall optimal outcomes for patients. These claims need to be assessed and supported by evidence. We have also heard that in some circumstances sponsors may not initially choose present existing evidence of additional benefits through submission of a full cost effectiveness analysis, and instead seek funding on the basis of not being inferior to a comparator.

##### Valuing long-term benefits

Both discounting and approaches to assessing uncertainty cause costs and benefits to be valued less the further into the future they are realised. This reflects the accepted societal preference for current over future benefits, and the reduced confidence in benefits and costs being realised the further into the future they are estimated to occur.

Relative to a health technology that delivers outcomes that are realised over the short term, the benefits of a health technology that delivers outcomes over a longer period are valued less. This does not significantly impact estimation of cost-effectiveness where costs are spread over the same time period as the benefits (e.g. where continued doses are required to maintain the benefit over time). This significantly impacts estimates of cost-effectiveness where the costs are upfront, and the benefits accrue over many years (such as for vaccines and gene therapies).

The effect of valuing long-term benefits less than more immediate benefits is that 2 therapies that deliver equivalent overall health outcomes at equivalent overall costs could have different estimates of cost-effectiveness if their costs are accrued over different time periods. The therapy with frontloaded costs would appear less cost effective than the therapy with costs spread more evenly over the period that benefits accrue into the future, notwithstanding equivalent overall costs and health outcomes.

The flexibility afforded by the absence of an ICER threshold and flexibility in discounting approaches and other aspects of economic evaluation enables the PBAC and the MSAC to account for where these variations impact cost-effectiveness estimates. For example, the PBAC considered a lower discount rate for the meningococcal B vaccine.

There is no explicit guidance on how this flexibility is exercised in PBAC or MSAC guidelines. This contributes to a perception that health technologies that deliver long-term benefits and have upfront costs are valued less than those that have costs that are spread over the time period that benefits accrue. The CHERE literature review found that approaches to valuing future costs and benefits in Australia are not significantly different to other countries although Australia’s discount rate is at the upper end of those that are used in comparable jurisdictions.

To the extent that HTA bodies in Australia and overseas do not explain how decision-making is modified to account for characteristics that alter estimates of cost-effectiveness, systems that, in the base case, make certain health technologies that have upfront costs and deliver long-term benefits appear lest cost effective, will be perceived as undervaluing these technologies.

#### Treatment of uncertainty in determining acceptable clinical and cost-effectiveness

Uncertainty about estimates of clinical effectiveness, cost-effectiveness, or cost is often cited as concern in PBAC and MSAC advice. In some circumstances, uncertainty is accepted, and funding is recommended. In other circumstances, uncertainty is not accepted, and sponsors are advised that a resubmission is required to address elements of the submission that create uncertainty.

Often confidence in estimates can be improved with changes to approaches used in a submission. This relates to the issue of resubmissions in that more optimistic assumptions supporting the cost-effectiveness of higher prices are often presented in first submissions and then revised in subsequent submissions. This is further discussed below in ‘Economic evaluation as price negotiation’.

In other circumstances, confidence in estimates cannot be improved due to deficiencies in the evidence base for certain technologies. This is acknowledged by HTA committees in their decision-making and in PBAC and MSAC guidelines. In these circumstances, committees will recommend approaches to manage this uncertainty after the health technology is funded.

Where there is uncertainty about the extent of use of a health technology, HTA committees will recommend risk-sharing arrangements to manage this uncertainty. Such arrangements are frequently used for new health technologies.

Where there is uncertainty about the clinical or cost effectiveness of a health technology, one option for HTA committees is to advise that a MAP or other arrangement that allows resolution of uncertainty after funding should be implemented. To date, such arrangements have been implemented infrequently and only where there are special circumstances of HUCN, and high potential added therapeutic value. This issue is discussed further in part 4 – managing uncertainty.

#### Incorporation of equity principles in valuing and/or decision-making

The guidelines for preparing submissions to the PBAC and the MSAC allow for applicants to present arguments that equity principles should be considered when determining whether the cost-effectiveness of a health technology is acceptable. For example, the PBAC guidelines allow applicants to present less readily quantifiable factors, such as matters related to equity, to be considered by the PBAC. Advisory committees consider this information and allow it to influence their decisions about whether or not a health technology should be recommended for funding.

While consideration of these factors, and the influence they have on decision-making, are documented in public summaries of committee considerations, it can be unclear to stakeholders to what extent they were factored into the decision-making. There is currently no explicit process for consideration of these factors and documentation of this consideration in public summaries.

#### Economic evaluation as price negotiation

The economic evaluation is the main determinant of the prices agreed between the Government and suppliers for health technologies funded under Australia’s funding and subsidy schemes. Because of this, the economic evaluation has become a proxy for negotiation of prices and other market access conditions.

Suppliers often present prices in economic evaluations for their first submission that are higher than what they will later accept in resubmissions. The cost-effectiveness of these early higher prices is often calculated using assumptions in economic models that are more optimistic than will be accepted by advisory committees.

Advisory committee acceptance of assumptions can also shift through resubmissions. They have, in some instances, shown a greater willingness to accept uncertainty in subsequent resubmissions than in early submissions.

New health technologies and new indications that are ultimately accepted by advisory committees as offering an improvement in efficacy or reduction in toxicity over all alternatives are almost never recommended for listing on the first submission. These are the health technologies that deliver the greatest improvement to health outcomes and include those that address longstanding unmet clinical needs. They are also the innovative technologies from which sponsors expect to make the highest levels of earnings, with high levels of return on investment.

Prior to the introduction of resubmission pathways, a decision to not recommend funding would significantly extend the time it would take for a health technology to be funded. Since their introduction in 2021, resubmission pathways have allowed advisory committees and sponsors to resolve issues more quickly.

Under current processes, the timeframe to funding remains several months longer for health technologies that are not accepted for funding the first time they are considered by an advisory committee. To address this issue would either require submissions to be acceptable and accepted the first time they are considered or issues to be resolved over a shorter timeframe.

We consider that for as long as economic evaluations in HTA have a price setting function, they will always be used as the proxy for price negotiation. We do not think it is realistic to expect that suppliers of health technologies could be compelled to put their best price and most conservative assumptions in their first submission, or that advisory committees should recommend funding irrespective of how optimistic assumptions are or how cost-ineffective initial prices would be.

We consider that options that allow for issues to be resolved over a shorter timeframe (see [section 2](#_Health_technology_funding_2)) or for uncertainty to be effectively managed after funding (see [section 4](#_Health_Technology_Funding_1)) are more feasible than options that would force the supplier or funder to accept funding conditions that they would otherwise consider unacceptable.

### Options

Many of the issues identified in relation to economic evaluations (e.g. recognition of elements beyond clinical effectiveness, cost-effectiveness, and financial impact) are addressed in other areas of this document. The following options focus on issues raised in relation to how the economic evaluation estimates the value for money of health technologies.

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| **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.   Refer: The operation of section 101(3B) of the *National Health Act 1953* – the selection of the comparator   1. In line 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 the above interpretation: 2. This could include a mechanism to differentiate different type of cost-minimisation submissions based on their proportional benefit. 3. 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.* |

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| **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 Government 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.  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 and the opportunity cost of medicines lost or not listed? |

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| **Valuing overall**  Conduct workshops to understand if and where it may be reasonable for HTA committees to accept higher prices for health technologies including:   1. in what circumstances 2. for what benefit 3. how much greater cost would be reasonable to secure that benefit 4. how confident do we need to be that we will be securing that benefit, and 5. 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.  Note: workshops could also be assisted through use of the explicit qualitative value framework proposed above (see [Develop an explicit qualitative value framework](#_Develop_an_explicit)). |

# Health technology funding and purchasing approaches and managing uncertainty

## Approaches to funding or purchasing new health technologies and managing uncertainty

This section should be read in conjunction with [*Funding and purchasing decisions and Managing Uncertainty (Paper 6)*](https://www.health.gov.au/resources/collections/health-technology-assessment-policy-and-methods-review-research-and-analysis-papers) and [*International health technology market approval funding and assessment pathways*](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-review-international-health-technology-market-approval-funding-and-assessment-pathways?language=en,) *(Paper 1).*

### Current state

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| ***A note on terminology…***  Conditional approval for reimbursement ​is an approach used to manage clinical, economic and financial uncertainty associated with funding health technologies. These schemes allow patients to access medicines that may not have received positive funding recommendations due to uncertainty inherent in the clinical evidence and/or the potential for considerable budget impact.  Such arrangements are referred to as managed entry agreements (MEAs), managed access programs or pathways (MAPs), and coverage with evidence development approaches (CEDs). These terms are sometimes used to describe approaches to manage one or other of clinical, economic or financial uncertainty or in relation to all three.  From paper: [International Health Technology Market Approval, Funding and Assessment Pathways](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-review-international-health-technology-market-approval-funding-and-assessment-pathways.pdf#page=8)[[10]](#footnote-11)  *Managed Entry: A conditional arrangement between a manufacturer and payer that enables earlier reimbursement of a health technology to address uncertainty in its performance or to manage its utilisation.*​  ​*CED: Early funding of a health technology conditional on gathering additional evidence to address the sources of uncertainty.*  From paper: [Funding and purchasing decisions and Managing Uncertainty](https://www.health.gov.au/sites/default/files/2023-12/hta-policy-and-methods-review-draft-paper-funding-and-purchasing-decisions-and-managing-uncertainty.pdf#page=9)[[11]](#footnote-12)  *Managed entry agreements (MEAs) are strategic arrangements between payers and sponsors, employed to ensure timely patient access to advanced healthcare treatments, particularly when there is uncertainty about their clinical or cost-effectiveness. MEAs are designed to address the inherent uncertainties surrounding the value, uptake and performance of emerging technologies through conditional or managed reimbursement.*  References to MEAs in this document should be taken to mean approaches to manage any type of uncertainty unless otherwise specified. |

International approaches to fund and purchase new health technologies vary widely, with differences in:​

* the depth and breadth of HTA processes ​
* the scope, role and remit of HTA organisations
* selection of technologies eligible for HTA review
* how HTA outcomes influence reimbursement decisions and pricing negotiations.​

Most countries that require HTA for government/insurance reimbursement of new health technologies undertake some variation of the following processes (with respect to the duration, order and combinations of these processes across countries):​

* market authorisation​
* product benefit assessment (e.g. clinical and economic evaluations)​
* recommendation / decision to fund ​
* price negotiation​
* post-HTA re-review / price reductions / disinvestment​.

Some jurisdictions (for example France) have implemented early access strategies to enable market entry prior to regulatory approval and reimbursement, through a streamlined process. Australia currently does not have a reimbursement pathway for medicines which have not received regulatory approval from the TGA.

Australia’s general approach to HTA funding and purchasing decisions is similar to most comparable countries. ​In Australia, price negotiations are confidential and are implicitly included in the HTA system. While the PBAC does not negotiate prices, it determines whether the proposed price for a medicine comprises acceptable cost-effectiveness. Following a positive PBAC recommendation, a PO package is lodged by the applicant to the Department before it is legislatively formalised by the Minister for Health. If the PBAC does not recommend a medicine for listing due to the proposed price, the applicant is responsible for proposing a more cost-effective price through a resubmission.

In relation to uncertainty, managed entry agreements (MEAs) between payers and sponsors are used internationally to facilitate timely patient access to advanced healthcare treatments, particularly when there is uncertainty about their clinical or cost-effectiveness. MEAs have been predominantly used for high-cost therapies where major health benefits are anticipated, with most examples being in oncology and rare diseases. Innovative approaches are being explored worldwide to refine MEAs for emerging therapies.

Australia currently operates several early MEAs or pathways, including the Managed Access Program (MAP) to provide access to products with HUCN but which have been identified by the PBAC/MSAC as having otherwise unacceptable clinical or economic uncertainty. PBAC and MSAC may also recommend that specific products be subject to financial-based MEAs (such as discounts, rebates, and volume/expenditure caps), or performance-based MEAs (typically Coverage with Evidence Development (CED) or payment-by-result/Pay-for-performance) within a risk-share arrangement context. MEAs can be considered within the usual PBAC/MSAC pathways and/or within the pathways of MAPs and the LDSP.

### What we heard from stakeholders

Throughout the consultation submissions, a key matter of concern for stakeholders in the HTA process has been the consideration of how best to manage uncertainty with respect to clinical outcomes, value for money (cost-effectiveness) and the overall financial impact (i.e. cost to patients, the Commonwealth, and state and territory governments) associated with healthcare technologies. Much of the input did not discuss financial and budgetary impact of funding health technologies explicitly with views regarding funding practices being expressed indirectly through comments on clinical/cost effectiveness and ‘price negotiation by resubmission churn’. A number of consultation submissions called for changes in how health technologies (including) advanced therapies) are evaluated and funded, particularly with respect to the consideration of costs relative to the uncertain clinical benefit, and how the dual clinical and economic uncertainty is managed.

Stakeholders noted that while there are approaches to addressing clinical and economic uncertainty within the system, particularly in the form of MAPs, they tend to be underutilised. Some stakeholders from the industry sector expressed the view that uncertainty was being used as a lever to lower prices, hindering efforts to focus on the plausible case. MAPs may be rejected by the global headquarters of the sponsor due to the low prices. Additionally, we have heard that sponsors may be hesitant to invest in CED programs across multiple countries.

Significant data collection infrastructure and governance also needs to be established to support program development. Stakeholders from the industry sector considered that this, coupled with a conservative stance in relation to how uncertainty is viewed in the current system, results in delays in reimbursement and pricing decision-making while steps to address clinical and economic uncertainty are agreed to throughout the HTA process.

### Issues

#### Managing uncertainty

We consider that a core issue for consideration in relation to this topic is whether revisions to the current funding, purchasing and/or uncertainty management arrangements could improve the balance between access, cost, timeliness and quality of decision-making associated with health technologies, to facilitate improved health technology access for Australian patients.

We note from the stakeholder submissions and HTA expert papers that across healthcare systems, frameworks to support healthcare payers in making funding and purchasing decisions for health technologies reflect the local policy, legislative and social contexts.

We have identified that certain common concepts and ideas from other countries around funding sources, financial/contract management and negotiation processes have been successful in improving timely access to HATV medicines for patients and may be transferrable and applicable to the Australian healthcare context.

In deliberating on the options that have been presented for further consultation, we note that for any such framework to be successful and responsive to the needs of consumers, proper identification, measurement and evaluation of stakeholder-agreed indicators with respect of access, cost, timeliness and quality of decision-making will be essential.

Opportunity for resolution following HTA committee consideration

We note that resubmission churn is a key factor in how long it takes to subsidise a health technology. While resubmission pathways introduced in 2021 have assisted in shortening the time in which advisory committees and sponsors are able to resolve issues, the time taken to subsidise health technologies that do not receive a positive recommendation at first consideration by an advisory committee is several months longer than those that do receive a positive recommendation at first consideration.

We consider that allowing for identified issues to be resolved over a short period of time may allow some submissions to become acceptable for positive recommendation by the HTA Committee.

We consider that an alternate HTA process sequence that may improve timely access to health technologies for HUCN would be for sponsors to receive early notice of negative HTA Committee recommendations due to clinical, economic, pricing and/or budgetary impact concerns. With the additional pricing-related context of the HTA committee recommendation in hand, sponsors would be provided a single time-limited opportunity to revise the submission to resolve identified concerns, such as via revisions to the price and/or use of MEAs in negotiations with the Commonwealth, or an opportunity to decline revisions and for the HTA Committee to finalise its negative recommendation.

The revised submission would then be reviewed by the HTA Committee as part of finalising the HTA recommendation, either resulting in a final positive recommendation, or a confirmed negative recommendation (and exit from the HTA process).

Budget impact implications of high-cost/high-impact health technologies

We note there is increasing international experience with alternative financial arrangements to address the practical problems and risks associated with high-cost/high-impact health technologies for health technology buyers and sellers. These include specific patient-level product warranties, annuity/mortgage payments and “subscription-style” bulk-funding programs. We note the use of alternative financial arrangements may in some cases improve negotiation outcomes and facilitate health technology access for patients (e.g. pricing and reimbursement arrangements being piloted in Western Europe as part of pull incentives to encourage the development and provision of antimicrobials). We would welcome feedback on the types of alternative financing tools and instruments that could be used in the Australian healthcare context.

In proposing the option to address budget impact implications of high-cost, high-impact health technologies for further consultation, we are aware of the significant complexity associated with how expenditure of Commonwealth funds and the relevant accounting standards that apply to such expenditures are governed by financial and public governance legislation, and also note that sponsor companies may have certain obligations under corporations and commercial law to report income and revenue generated through product sales. It is therefore likely that, if this option was included in final HTA Review recommendations, extensive additional consultation outside of the review would be required with stakeholders and respective Commonwealth agencies (principally the Commonwealth Department of Finance) to identify and address additional practical implementation risks associated with this option.

#### Recognising competition between different health technologies

Some new health technologies are so similar in effect to alternatives that are already funded, that they offer minimal advantage either in terms of improved efficacy and safety, or as an alternative treatment option that may deliver better outcomes for some patients. When funded, these health technologies compete with existing products for market share. Ordinarily the public benefits when this occurs in a market because it drives competition between suppliers on price and delivers lower prices.

This competition is not recognised in decisions by the Commonwealth to fund health technologies in Australia. New health technologies of this type are funded at the same cost as existing ones when in an unsubsidised market they would need to be marketed at a lower price.

#### Pricing offer and negotiation guidance framework

We note that stakeholder feedback previously provided during the HTA Review that one of the drivers of uncertainty and delay in health technology access is the lack of certainty in whether (and how) the incremental additional clinical benefit of a health technology is accounted for (as part of “value considerations”) in POs and price negotiations between sponsors and healthcare payers.

We also note that the price negotiation process by definition creates a dynamic tension where:

* healthcare payers are incentivised to seek the lowest possible purchase price to meet legislative and social obligations to ensure efficient use of public resources
* health technology suppliers are incentivised to sell their product at the highest possible purchase price to maximise revenue from intellectual property and meet shareholder benefit expectations, and
* patients may be waiting for the negotiation process to finalise before subsidised access to health technologies are available.

In our deliberations, we noted that the amount of negotiation time required to close the pricing gap between payer and supplier for certain health technologies may result in an opportunity cost that is inefficient and does not yield the best balance of outcomes for all stakeholders. For individual patients, delays in access may have profound negative consequences.

We noted information in the HTA expert papers, that a number of international healthcare systems have sought to provide more explicit guidance to health technology sponsors in respect of pricing expectations and boundaries that are influenced by the clinical benefit of a health technology (as identified in a HTA evaluation), in order to facilitate faster price setting and negotiation processes. Examples internationally have included:

* expectations and/or requirements for lower prices for health technologies that are evaluated on a cost-minimised basis and provide only marginal (or no) added clinical benefit compared to existing treatments (such as the arrangements currently in place in Germany)
* tiered rebate/reimbursement arrangements, with rates that are linked to the incremental therapeutic benefit of a health technology compared to other available treatments (such as the arrangements in place in France and under consideration in Canada)
* specific pricing modifiers linked to certain priority criteria of clinical/health equity significance (such as the arrangements in place in Japan).

#### Re-review of health technologies

We note that an underreported and under-discussed aspect of funding and purchasing decision-making by healthcare systems is the concept of disinvestment (i.e. the deliberate and systematic reduction of funding for a health technology of questionable or comparatively low value). In deliberations on this matter, we noted examples of passive and active disinvestment frameworks in Western European healthcare systems, including price adjustment mechanisms, clinical guideline reviews influencing service provider and payer behaviours, and explicit health technology re-review programs.

We acknowledge that disinvestment can be a complex issue for stakeholders due to a range of expectations and sensitivities, particularly in respect of perceived loss of clinical choice and the lack of clarity in how disinvestment decisions are made in practice. However, we also consider that a mature and well-administered healthcare system needs to be supported with funding and purchasing policies that facilitate the review of health technologies throughout their utilisation and subsidisation lifecycle, as new evidence on clinical practice and SOC over time may justify the reallocation of finite resources to improve and support patient health outcomes.

We note the Australian system has elements of disinvestment advice provision and mechanisms in place via the current drug utilisation subcommittee and post-market review arrangements, as well as statutory price reductions, and reference pricing. We also note that disinvestment occurs through clinical decisions, some stakeholders consider, based on the available information, that a more established program could improve on current arrangements, and provide a clear mechanism for stakeholders to consult, discuss and agree on disinvestment recommendations in a predictable and rigorous manner.

#### Time-limited funding to manage uncertainty

We note a number of Western European examples where special funding programs have been established for the purposes of providing access to promising health technologies that meet a clear unmet need (when compared to standard funding, and reimbursement and commissioning approaches for health technologies). These funds typically have specified qualifying conditions and are restricted to certain health technologies and/or clinical indications (the UK Cancer Drugs Fund and Innovative Medicines Fund are examples).

We note the general outcomes of these funding and purchasing arrangements is to either:

* bring forward health expenditure (and patient access) that likely would have been incurred eventually after a HTA evaluation that provided a positive recommendation, and/or
* provide a capped source of new funding for health technologies of high clinical significance and relevance to patients to improve health equity, but that also have a measure of clinical, economic and/or financial uncertainty in respect of whether such funding allocations are an efficient use of health expenditure.

We acknowledge that the funding and governance structure of a special funding program would require extensive consultation beyond the scope and remit of the HTA Review and be subject to a decision of government. However, we consider that initial provision of stakeholder feedback and advice (if received) on the types of program design criteria (as articulated in the option below) may be of useful to the Government (if a final committee recommendation includes elements of this option and the Government believes it merits further consideration).

We also note a small number of G7 (Group of 7) healthcare systems (Germany and Japan) have been structured to support subsidised patient access to a broad range of health technologies at free market prices for a temporary period, prior to HTA evaluation (and subsequent price negotiation). While we considered the portability and applicability of similar pricing and negotiation arrangements for the Australian healthcare context, we also noted that those systems operate under very different healthcare financing structures (being private health insurance schemes operating within a defined regulatory framework) and have distinctly different social policy and legislative perspectives regarding the role and function of the government in price setting and negotiation compared to the Australian healthcare system, which makes the transferability of those arrangements more complex.

#### Guidance on and policy arrangements for MEA instruments

We note a range of stakeholder submissions have discussed the potential for managed access arrangements to support timely access to health technologies for patients, and how the current MAP in its current design could be improved to support this objective.

We also acknowledge the findings from the House of Representatives Standing Committee Inquiry ([the Inquiry](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report)) report, [The New Frontier - Delivering better health for all Australians](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report) (The New Frontier report), and the [Australian Government response](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Government_Response) to the report in respect of the recommendations on managed access arrangements.

In deliberating on these matters, we note that all of the MEAs instruments utilised internationally are also used in some form as part of current approaches to deed negotiation between the sponsor and the Commonwealth, but that existing awareness of these arrangements is variable among sponsors.

### Options

##### Funding and purchasing

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| **Alternative option 1:**  In conjunction with options for proportionate assessment of cost-minimisation submissions (see [Proportionate appraisal pathways](#_Options_–_Proportionate)), 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](#_Options_–_Proportionate))](#_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. |

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| **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 [Health Technology Funding and purchasing approaches and managing uncertainty](#_Health_Technology_Funding_1)) 2. resolving issues in submissions that prevent positive recommendations being made [(see early resolution options)](#_Options_–_Proportionate) 3. addressing lack of incentive for developing health technologies in certain areas [(see Health technologies that address antimicrobial resistance)](#_Health_technologies_that) |

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| **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:   1. all health technologies submitted for HTA evaluation; 2. health technologies submitted for HTA evaluation on a cost-minimisation basis; or 3. 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) |

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| **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. |

##### Managing uncertainty

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| **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:   * Early identification and nomination via horizon scanning and/or designation on a Priority List of HUCN conditions. * 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) * Requirement for parallel TGA/HTA Committee submission lodgement as part of a broader overall approach to support timely recommendations. * Approach undertaken by the applications and evaluation that:   + 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   + 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. |

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| **Revised guidance on the uses of different managed entry tools**  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 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. |

## Health technologies that address antimicrobial resistance (AMR)

### Current state

In recent years, the healthcare implications associated with the rise in AMR have gained prominence in health policy dialogue, as seen by recent statements by the G7 nations expressing concerns regarding the population health, social and economic consequences associated with AMR.[[12]](#footnote-13)

Of relevance to this review are matters in respect of the availability of antimicrobial health technologies in the Australian market, and the current HTA processes and policies that are applied in the evaluation of such health technologies for market authorisation and public subsidy.

As noted in the commissioned [literature review on international approval, funding and assessment pathways](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-review-international-health-technology-market-approval-funding-and-assessment-pathways?language=en):

“*There is currently no special pathway for the reimbursement of antimicrobials in Australia. The PBAC guidelines do highlight, however, that submissions for new antimicrobials must consider the ‘General principles of antimicrobial use’ contained in Therapeutic guidelines: antibiotic and principles proposed by the Joint Expert Advisory Committee on Antibiotic Resistance for consideration of the target population”.*

Across the [research papers commissioned by the Reference Committee](https://www.health.gov.au/resources/collections/health-technology-assessment-policy-and-methods-review-research-and-analysis-papers), the following observations in respect of AMR and evaluation and funding for antimicrobials in the international context were noted:

* Most countries do not have a special pathway for the reimbursement of antimicrobials, although certain countries allow limited exemptions and flexibility in the reimbursement process for antimicrobial products.
* HTA agencies have historically had scarce guidance on evaluating antibiotic agents, antimicrobial agents, communicable diseases, and infectious diseases.
* In Germany and France, certain antimicrobials are exempted from HTA (as part of a policy that presumes therapeutic benefit for designated products) and may be afforded additional price negotiation flexibility and/or excluded from standard reference pricing arrangements.
* In the UK, a recent pilot HTA process by NICE and National Health Service (NHS) England trialled a delinked price-volume payment model for 2 antimicrobial products, as well as an alternative HTA process utilising additional dimensions of value specific to antimicrobials (spectrum, transmission, enablement, diversity, insurance value – (Spectrum, Transmission, Enablement, Diversity, Insurance Value (STEDI)) combined with standard HTA dimensions in the UK context (such as clinical effectiveness, costs, safety, etc).
* In Sweden, a partially price-volume delinked guarantee of supply arrangement was tested to determine if such financial support (as a pull mechanism) would make additional antimicrobials of significant medical value available in the Swedish market.
* In the US, the FDA may grant special “qualified infectious disease product” designations which provide additional market exclusivity extensions specific to the designated antimicrobial product.

The research papers also noted the following HTA process and policy implications that can arise when evaluating antimicrobial products:

* NICE noted as part of its documentation supporting the antimicrobial HTA pilot that:
  + There can be difficulties in defining the relevant population and subgroups for antimicrobials, as market authorisation may be focused on pathogens rather than clinical indications.
  + In determining the population for antimicrobial utilisation, consideration should be given to the setting of antimicrobial use (community, hospital or restricted to intensive care use), as the rate of infections and transmission dynamics will differ based on the setting.
  + The benefits of antimicrobials can extend beyond the patients treated, to the wider population, so the perspective of the HTA evaluation needs to be explicit.
  + As antimicrobials may be used for a wide range of different indications, there can be a variety of comparators based on the infection site, pathogen, and mechanism of resistance, and whether the treatment is used in the microbiology-directed or empiric setting (i.e. after testing the susceptibility of the pathogen, or on the basis of clinical suspicion of the pathogen and its mechanism of resistance).

A number of recent academic papers have also argued for revisions to HTA guidance documents to emphasise the consideration of community externalities associated with antimicrobial agents and other infectious diseases, including:

* accounting for reduced transmission rates
* costs of treating resistant cases
* QALY gained from avoiding infection, and
* performing sensitivity analyses on different levels of resistance.

### What we heard from stakeholders

The Reference Committee noted that only a limited number of stakeholders directly addressed matters in respect of AMR and antimicrobial access and availability during the initial consultation. Much of the stakeholder feedback received can be summarised in the following observations from the Australian Antimicrobial Resistance Network submission to the HTA Review:

*There is a widely acknowledged market failure that exists for novel antimicrobials that makes investment in their development and commercialisation loss making. This market failure exits for several reasons;*

*1. Uptake of novel antimicrobials is slow*   
*2. Constrained hospital budgets*   
*3. A lack of rapid point of care diagnostics*   
*4. Traditional reimbursement systems undervalue novel antimicrobials*   
*5. Clinical value is difficult to demonstrate*   
*6. High cost of product launch*   
*7. Lack of regulatory pathways and incentives specific for antimicrobials*

*Australia has the additional challenge of being a small market, and this, combined with the high cost of registration here, limits the availability of new antibiotics, restricting access to the best therapies.*

*Current reimbursement methods, where payments are linked to sales volume, do not recognise the broader value that antimicrobials provide to society as crucial lifesaving, and health system-enabling medicines.*

### Issues

We note the evidence and information provided that point to the core problem statement, being that the current general approach to the HTA evaluation and HTA Committee recommendations in respect of antimicrobial health technologies may not be sufficiently flexible to support timely access and appropriate levels of availability to address population health needs.

We also acknowledge that, given the market failure and the broader challenges in respect of antimicrobial product availability (from initial clinical development through to eventual health system access), and the dynamic tension between economic, social and health system objectives with respect to the use and stewardship of antimicrobial products, it is likely that reform options and recommendations proposed will need to be considered in conjunction with other policy measures that are beyond the scope of this Review.

Supporting this view, we acknowledge that the [Government’s response to Recommendation 27](https://www.health.gov.au/resources/publications/inquiry-into-approval-processes-for-new-drugs-and-novel-medical-technologies-in-australia) in The New Frontier report states:

*the Department of Health and Aged Care has commenced work towards identifying and scoping potential funding mechanisms and economic models that could be considered in the Australian context to incentivise the discovery of novel antibiotics and bring them to market in Australia*.

The Reference Committee notes the high interest in ensuring appropriate diversity and volume of antimicrobial health technologies are available to both current and future Australian patients, and notes that in some cases, cost burdens associated with lodging reimbursement submissions may be a barrier to entry for smaller companies (noting submissions received suggesting costs of up to $1 million in evaluation fees per product).

The Reference Committee acknowledges that the current PBAC cost recovery framework permits the granting of fee waivers and exemption for products of high public interest as defined in regulations, and where payment of fees would make a submission financially unviable for a potential applicant.

The Reference Committee notes the development and testing of alternative approaches to HTA for antimicrobial health technologies in overseas jurisdictions, including modifications to PICO criteria definition, evidence evaluation approach and dimensions of value considered when evaluating these health technologies. Of particular note is the trend by researchers, health policy agencies and HTA agencies towards acknowledging the broader indirect population health protection implications associated with the proper use of antimicrobial health technologies, and the positive longer-term health system effects that may come with the availability of a broader suite of narrow-spectrum antimicrobial health technologies (which help in managing future AMR risk).

As the [Government’s response](https://www.health.gov.au/resources/publications/inquiry-into-approval-processes-for-new-drugs-and-novel-medical-technologies-in-australia) to the [New Frontier report](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report) states that the Department has commenced work towards identifying and scoping potential funding mechanisms and economic models to incentivise market availability of antimicrobial products in Australia4,

Similarly, the Reference Committee also acknowledges alternative payment and reimbursement reforms being tested internationally, and notes the keen interest expressed by some stakeholders in delinking price and volume for antimicrobial products given the balancing public health policy approach to product stewardship to address AMR in the long term.

The Reference Committee considers that, given the different AMR patterns in local health contexts, any options in respect of alternative payment and reimbursement reforms for antimicrobial products will need to be relevant to the Australian health system context (including current Commonwealth and jurisdictional funding structures for health technologies), while also being broadly consistent with global strategies providing incentives to bring antimicrobial products to market.

### Options

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| **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. |

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| **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. |

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| **Funding and reimbursement-related changes to support availability of antimicrobials (possible pull incentives)**  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. |

## Understanding the performance of health technologies in the Australian setting

This section should be read in conjunction with [Optimised real world evidence to support health technology assessment in Australia (paper 7).](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-optimised-real-world-evidence-to-support-health-technology-assessment-in-australia?language=en)

### Current state

Where a decision is taken to resolve uncertainty about the clinical and cost-effectiveness of health technologies after they are funded, information on the health outcomes of patients receiving the health technology needs to be collected. As identified in previous sections, to date there have only been a small number of examples where this has occurred in Australia.

Decision-making across the health technology pipeline is iterative. At each point, decision-makers must assess whether the existing evidence addresses their uncertainties, fully or partially. Where significant uncertainty exists, new evidence must be generated; every time new information is generated, evidence gaps are narrowed or closed and/or new questions arise.

RWE, generated through the analysis of RWD, plays an important role in supporting the evidentiary needs of decision-makers across the health technology pipeline, including for market authorisation and subsidy approvals, as part of HTA.

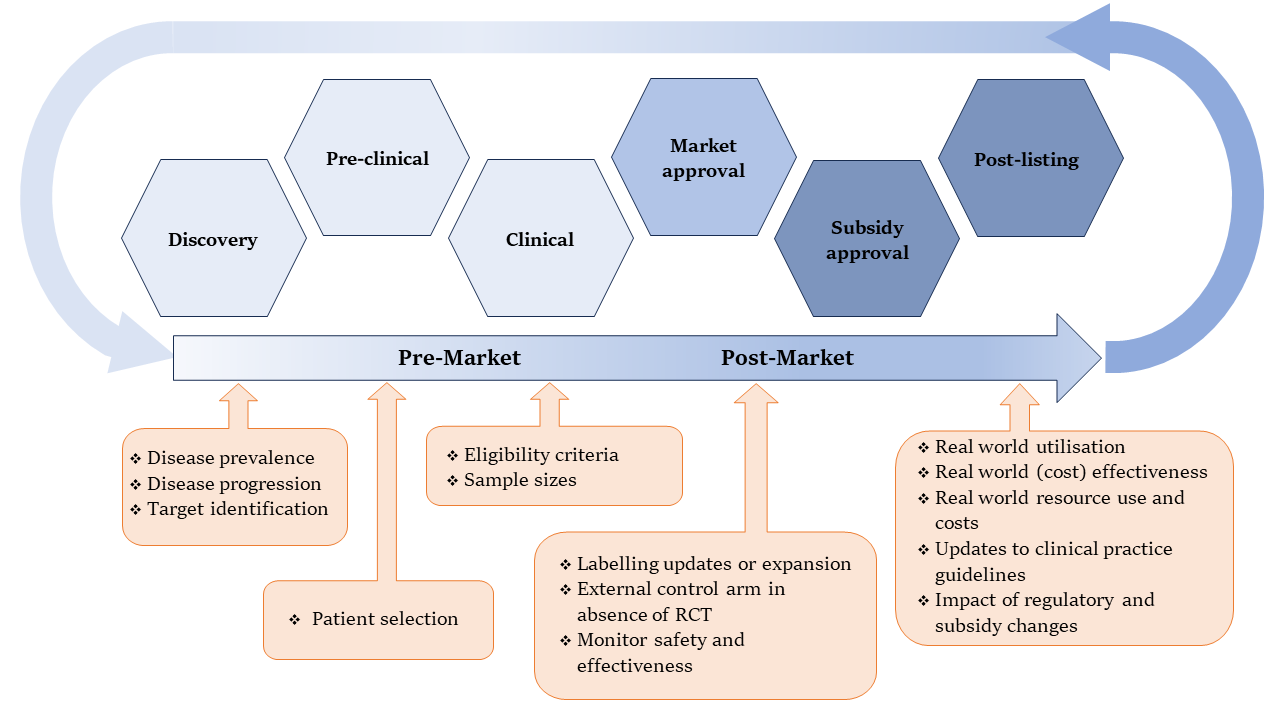
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| ***A note on terminology…***  RWD is defined by the International Network of Agencies for HTA as data collected during the routine delivery of healthcare, outside of clinical trial conditions. Other agencies and groups have more expansive definitions that also include data collected routinely across all aspects of health and social care, through disease and health technology-specific registries and directly from patients through digital platforms.  Key sources of RWD in Australia which may be used to inform HTA include:   * administrative data * clinical data – electronic health records * registries (clinical and disease registries) * surveys * molecular / diagnostic data * mobile data collected by a third party outside of formal healthcare delivery * case reports * social media.   RWE is the clinical evidence regarding the usage and potential benefits or risks of a health technology derived from analysis of RWD. While the recent interest in RWE has resulted in several frameworks and guidance documents describing practices for its collection and evaluation, definitive guidance is undermined by a lack of a narrow or clear definition of RWE as it applies to HTA. |

RWE generated from RWD can provide policymakers with a more comprehensive understanding of the risks and benefits of health technologies in routine clinical care, which often differ to outcomes observed in RCTs.

With the growth in access to, and linkage of, RWD across many disease areas and clinical settings, opportunities for RWE to both enhance and complement RCT evidence and support the evidentiary needs of decision-makers across the entire pipeline are evolving. As the capacity to link heterogeneous data at the person level has increased, it is now more feasible to bring together disparate RWD collections, thus enhancing the uses of these data to support HTA.

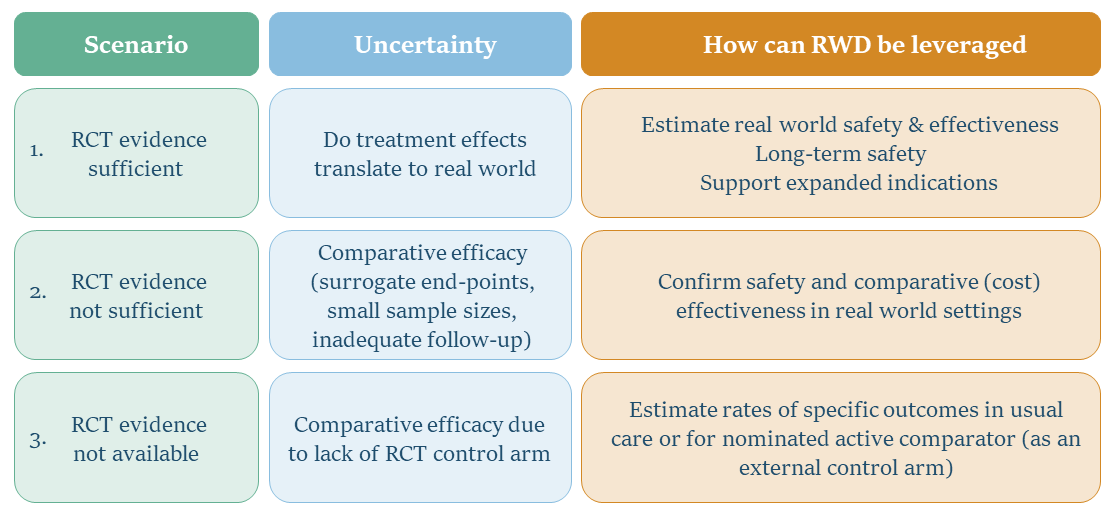
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| In [paper 7 Optimised real-world evidence to support health technology assessment in Australia](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-optimised-real-world-evidence-to-support-health-technology-assessment-in-australia?language=en), MI-CRE provided an overview of how RWD and RWE have played an increasingly significant role in supporting decision-making across the health technology pipeline, including:   * **HTA (Subsidy Approval):**   + To estimate real-world comparative (cost) effectiveness of health technologies relative to SOC or existing treatments.   + To generate inputs for (cost) effectiveness analyses by generating insights about real-world resource use and associated costs. * **HTA (Post-Listing):**   + To input into clinical practice guidelines and benchmark guideline-recommended versus actual care.   + To support continuous assessment of real-world use, safety, (cost) effectiveness and economic impact of health technologies in diverse populations and complex, dynamic healthcare settings. This includes performance monitoring for health technologies subsidised under provisional arrangements (e.g. managed entry or pay-for performance) and in the context of newly listed alternative therapies (e.g. post-market review).   + To evaluate the impact of subsidy changes on real-world use and outcomes of specific health technologies.   + To empower patients to make informed treatment choices using outcomes of patients with similar characteristics. |

###### Figure 7 Applications of RWD across the health technology pipeline



Additionally, RWD/RWE may be further to supplement evidence from RCTs to support claims of (cost) effectiveness, as showing in figure 1.

###### Figure 8 Scenarios where RWD can generate comparative treatment effects in HTA



### What we heard from stakeholders

Many stakeholders expressed a desire for greater use of RWE to support the assessment of clinical and cost-effectiveness of health technologies throughout the HTA lifecycle including after health technologies are funded. Stakeholders also expressed a desire for improved guidance and clarity on how RWE could be used for this purpose. These issues are further discussed in the section on clinical evaluation.

There was a common sentiment from stakeholders that existing sources of RWD could be better used to generate the RWE to support funding decisions after health technologies are funded.

### Issues

Australia does not systematically evaluate whether the subsidised health technologies work as well in practice as expected from the original subsidy assessment.

The Reference Committee has observed that Australia’s processes and systems for collecting the information needed to determine the clinical and cost-effectiveness of health technologies after they are funded are in early development.

The committee has heard that the experience from the small number of health technologies that have been funded in Australia CED-type approaches is that it remains difficult to establish how well the technology is performing. See example below:

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| **Chimeric antigen receptor T cell therapy (CAR-T) tisagenlecleucel (TIS)**  MSAC supported funding of TIS in 2019 for the treatment of acute lymphoblastic leukaemia in paediatric and young adult patients.  Public funding was supported on the basis that clinical, economic and financial uncertainty would be resolved after it was funded though:   * Ongoing data collection in a registry * A full review of clinical effectiveness, cost-effectiveness and budget impact * A pay for performance arrangement, and * Financial caps.   The review was considered by the MSAC in 2023. MSAC considered the available evidence did not fully address the clinical, economic and financial uncertainties that existed when it initially supported public funding. MSAC also considered the review did not adequately address MSAC’s previous concerns about the uncertainty of clinical outcomes beyond 12 months for patients treated with TIS.  The MSAC considered that the data were incomplete and inadequate to inform whether the expected longer-term effectiveness and safety outcomes from TIS are occurring in practice. |

#### Limited availability and feasibility of high quality RCT evidence for emerging technologies and small patient populations

Notwithstanding this experience, the Reference Committee appreciates that the need for CED approaches is likely to increase.

Many new and emerging health technologies are likely to be used in highly select and very small patient populations (e.g. rare and ultra-rare diseases), with poorly characterised natural disease history, uncertain epidemiology, heterogenous phenotypes, and lack of diagnostic accuracy. As such, the key evidence uncertainties will result from insufficient or unavailable RCT evidence.

RWD and RWE may be used to supplement RCT evidence to inform decision-making regarding subsidy of health technologies proposed for use in Australia and reduce uncertainty. An increasing role for RWE and RWD is to gain more information on the outcomes associated with funded technologies, including those provisionally listed, particularly where there is uncertainty around long-term outcomes or the specific patient population. Having systems in place to enable the timely and accurate collection and reporting of utilisation and outcome data associated with provisionally listed technologies will be critical to the establishment of any CED or provisional listing access arrangement.

#### Quality of RWD and RWE

The acceptability of RWE for HTA is often hindered by questions relating to quality. RCTs are considered the highest quality evidence in demonstrating the efficacy of a health technology as they are designed to address a specific study hypothesis or question; randomisation is used to ensure that bias and confounding is minimised; data are collected purposefully; and data curation is highly regulated. In contrast, studies leveraging RWD, where data is commonly collected in real-world clinical settings, are subject to bias and confounding and may not have complete capture of all necessary information.

The quality of RWE is multifactorial: it relies on the quality of the underlying data (provenance, reliability and missingness), the quality of the methods used to analyse those data (appropriate study design and analytic methods to control for bias) and the quality of the question itself (data fit for purpose to address the question). Issues relating to data quality may be considered in terms of relevance (availability, representativeness), and reliability (accuracy, completeness).

The Reference Committee also appreciates that methods for minimising bias and confounding in studies using RWD are becoming more sophisticated and may help to reduce uncertainty when these data are used to answer questions about clinical and cost-effectiveness.

#### Absence of a coordinated national approach to collecting RWD on health outcomes

The Reference Committee considers that one of the barriers to implementing CED approaches is the absence of data infrastructure and standards that would enable health outcomes data to be collected in a way that supports assessment of clinical and cost-effectiveness.

The Reference Committee appreciates that there are existing approaches for collecting health outcomes data used in state and territory hospital systems but notes that these are not coordinated, the data are not widely available for use to evaluate health technologies funded under Australia’s funding schemes, and that they may not be collected in a way that would enable questions about clinical and cost-effectiveness to be answered.

### Options

The Reference Committee believes there are significant opportunities to maximise the value of RWD and RWE for HTA in Australia in coming years. It would like to seek feedback on the following options based on the roadmap provided in the commissioned expert paper, [*Optimised real world evidence to support health technology assessment in Australia (paper 7*](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-optimised-real-world-evidence-to-support-health-technology-assessment-in-australia?language=en)*)*.

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| **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. |

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| **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. |

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| **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. |

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| **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. |

It should be noted that these structures would take a number of years to effectively establish, and options for collection of outcome data associated with the use of any provisionally listed health technologies is also required in the short term in order to inform decisions around continued subsidy.

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| **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. |

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| **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. |

# Futureproofing Australia’s systems and processes

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| This section should be read in conjunction with [Horizon Scanning and Early Assessment](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-horizon-scanning-and-early-assessment.pdf) and [International Health Technology Market Approval, Funding and Assessment Pathways](https://www.health.gov.au/sites/default/files/2023-10/hta-policy-and-methods-review-draft-paper-review-international-health-technology-market-approval-funding-and-assessment-pathways.pdf) |

## Proactively addressing areas of unmet clinical need and gaps in funded access

### Current state

Health technologies are funded in Australia in response to applications from sponsors to fund access in Australia. This is necessary because health technology companies usually hold the scientific data and other information necessary to inform HTA Committee’s consideration. Further, funding arrangements will impact health technology companies’ decisions to market a product in Australia.

HTA in Australia is reactive. It reacts to submissions being made from sponsors. We currently do not have proactive processes for identifying areas of unmet need and potential treatments being developed, or that have been developed that could meet those unmet needs.

### What we heard from stakeholders

Stakeholders suggested that Australia adopt a more proactive approach to identifying therapies that address unmet needs such as is undertaken by the CADTH and the National Institute for Health and Care Research Innovation Observatory (NIHRIO) in the UK.

### Issues

We consider that for as long as we are not identifying where we have unmet needs, and potential therapies that may address them, we will not be in a position to assess how well our system is meeting the needs of Australians. We have heard that Australia should undertake more formal horizon scanning and ways to repurpose existing medicines. We consider that identifying unmet needs is a precursor to identifying health technologies that could potentially address them.

### Options

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| **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](https://www.closingthegap.gov.au/national-agreement) 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. |

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| **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 peoples 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*](#po_HS) *for further information and additional preferred options considered by the Reference Committee relating to horizon scanning.* |

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| **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. |

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| **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 [Health Technology Funding and purchasing approaches and managing uncertainty](#_Approaches_to_funding_1))   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. |

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| **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). |

## Horizon Scanning

### Introduction and current state

The Reference Committee acknowledges the keen interest of stakeholders in the topic of horizon scanning as expressed in stakeholder consultations and presentations to the HTA Review. The Reference Committee notes the importance of establishing a common baseline understanding of what horizon scanning is (and its intended purposes) and therefore encourages stakeholders to consider the accompanying [paper 2 on Horizon Scanning and Early Assessment](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-horizon-scanning-and-early-assessment?language=en) when providing feedback on the options and concepts discussed in this chapter.

In brief, horizon scanning is defined in the [HTA Glossary](https://www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-glossary) as “the systematic identification of health technologies that are new, emerging or becoming obsolete and that have the potential to effect health, health services and/or society”.

Related concepts described in the HTA Glossary and broader literature are:

* early awareness and alert systems, which are defined as “*a system that aims to identify, filter and prioritise new and emerging health technologies, or new uses of existing interventions; to assess or predict their impact on health, health services and/or society; and to disseminate information”*
* early warning systems, defined as “a stable unit with reliable connections and sources which aims to: identify new technologies that have the potential to make a large impact on health services; filter and prioritise these technologies to select those most likely to have an impact on health, services and budgets; and assess that impact”.

For the purposes of this 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.

Australia currently does not perform any horizon scanning activities in healthcare at the national level, although it has performed limited horizon scanning in the past through the HealthPACT and subsequent advisory committee arrangements, which reported to the former Australian Health Ministers’ Advisory Council) prior to its dissolution in 2020.

### What we heard from stakeholders

Many stakeholders expressed concern that Australia does not have localised horizon scanning arrangements at the national level and reflected on the fact that comparable healthcare systems either have localised horizon scanning programs (e.g. CADTH and NIHRIO) and/or participate in cross-border horizon scanning collaborations (e.g. International Horizon Scanning Initiative (IHSI).

The Reference Committee noted that stakeholder reflections on the purpose of horizon scanning could be grouped broadly into one or more of the following areas:

* identifying future products and technologies in therapeutic areas
* gathering patient insights in respect of health technologies of interest
* identifying gaps in knowledge and data relevant to a given health technology
* informing assessment pathways (and necessary flexibility/modifications) relevant to future health products/technologies (especially for cell/gene therapies)
* informing health system resourcing and preparedness decisions necessary to support the introduction/adoption of new health technologies.

### Issues

Similarly, the stated purposes of horizon scanning arrangements overseas was set out in [paper 2’s](https://www.health.gov.au/resources/publications/hta-policy-and-methods-review-draft-paper-horizon-scanning-and-early-assessment?language=en). These purposes were broadly categorised into the following areas:

* identification of pricing for health technologies to inform reimbursement decisions or negotiations
* assisting funders and suppliers to determine appropriate assessment pathways for health technologies
* directing regional health service planning
* providing advance notification about health technologies to health service policy bodies.

Additionally, for most agencies, horizon scanning generally followed a process consisting of:

* topic identification
* filtration of topics, and
* selection (prioritisation) for further attention.

The Reference Committee also noted the trend towards horizon scanning collaborations internationally due to the resource intensity of horizon scanning arrangements and noted that alignment between jurisdictions on the goals and purposes of horizon scanning were essential to produce meaningful outputs.

In reflecting on the information presented during the HTA Review, the committee noted circumstances where a national horizon scanning program that supports HTA activities may have improved the quality and speed of HTA recommendations and health technology implementation arrangements, facilitated clinician and patient accessibility to certain health technologies and improved resulting patient health outcomes.

Case examples discussed included the initial HTA evaluation and implementation of recommendations for the provision of advanced therapies such as CAR-T (cell therapies) and gene therapies.

Further, in considering the programs and other international examples discussed in the research paper and flagged by stakeholders, it is clear to the committee that, should horizon scanning arrangements be reintroduced, identifying the following baseline characteristics for such a program are essential to ensure its success in the Australian context:

* **scope** (technology type and time horizon to be horizon scanned)
* **audience** (intended recipient of horizon scanning outputs, such as healthcare payers, decision-makers or patients)
* **purpose and objectives** (what is horizon scanning to be used for (e.g. improving patient access to scanned technologies, supporting information exchange between healthcare providers)?
* **process and methods** (including matters of governance, decision-making checkpoints, as well as information input source considerations, as well as the participants in, and commissioners of, horizon scanning activities)
* **outcomes/outputs** (i.e. the content, and how that content is presented to the target audience for their consideration).

### Options

Establishment of horizon scanning programs to address specific informational needs within HTA and the health system.

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| **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 high-cost, 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 MSAC,) to support forward planning and priority setting. (see  [State and territory government collaboration in HTA](#_State_and_territory)) 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. |

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| **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 leverage off patient and clinician communities, 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](https://static1.squarespace.com/static/62ebb08a9ffa427423c18724/t/64467ee62c9e8f38067d2352/1682341610670/National-Agreement-on-Closing-the-Gap-July-2020.pdf), 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)* |

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| **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: 2. 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 3. understand the potential implications for the Commonwealth from the introduction of these advances in terms of resources, systems and processes. 4. If this mechanism is not meeting its objectives, investigate alternative mechanisms to achieve these objectives in collaboration with industry (including other mechanisms (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). |

## Environmental considerations in HTA

### Introduction and current state

Globally, healthcare currently contributes 5% of all greenhouse gas emissions and decarbonisation is urgently needed. In Australia and other high-income countries, manufacturers of health technology products are making commitments to decarbonise their production processes as part of national efforts to achieve net zero emissions. Cochlear Ltd and Sonic Healthcare have both committed to achieving net zero direct and indirect emissions by 2050, while in relation to Scope 1[[13]](#footnote-14) and 2[[14]](#footnote-15) emissions, Cochlear has committed to net zero by 2030 and Sonic Healthcare has committed to a 43% reduction against 2005 levels by 2030. These commitments reflect the expectations of shareholders and capital markets that health technology manufacturers contribute responsibly to global efforts to mitigate the threats to people and the planet posed by climate change. As part of these efforts to decarbonise health technology manufacturing, efforts are under way to not only measure greenhouse gas emissions at a company level, but to also measure the emissions associated with individual health technology products. This is best done via ‘process-based life cycle assessment’, which maps environmental impacts associated with each stage of the product’s life cycle, including raw material extraction, manufacturing and assembly as well as use and end of life (‘cradle to grave’). Environmentally Extended Input Output analysis, an alternative method which uses only financial data to estimate footprints, is not appropriate for this purpose.

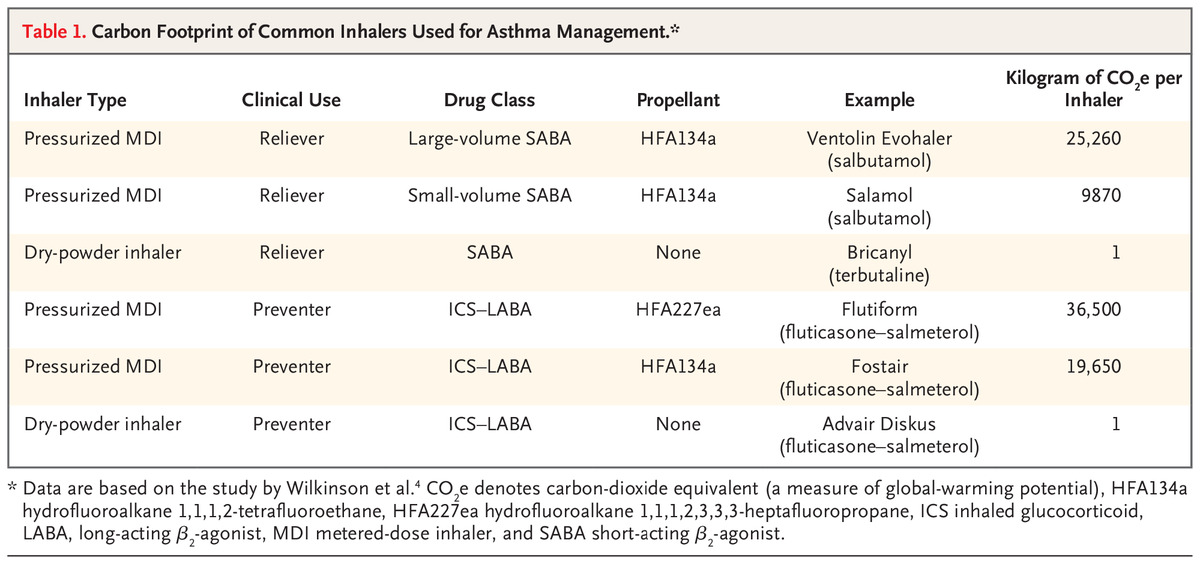
### Issues

**Benefits of reporting greenhouse gas emissions associated with health technology products**

There are two key benefits associated with measuring and publicly reporting the greenhouse gas emissions associated with health technology products.

The first benefit is that reporting emissions embodied in health technology products can support low carbon choices in clinical decision-making. While clinicians should always prescribe the most clinically beneficial option, in many scenarios there are a range of competing products that are recognised to be broadly clinically equivalent for most patients. In such cases, knowledge of embodied emissions could enable clinicians to prescribe (and patients to ask for) the most environmentally friendly option. An example includes the choice of respiratory inhaler to treat asthma and other respiratory conditions (see Table from an example study below).[[15]](#footnote-16)

The second benefit is that this information can be used to inform health technology assessment approval and reimbursement decisions. In cases where a new technology has largely equivalent health benefits and costs to existing technologies, but a significantly larger emissions footprint, this could create a case for declining a reimbursement application. In cases where a new technology has equivalent health benefits and higher costs than existing treatments but a much lower emissions footprint, this could create a case for approval when such a decision might not otherwise have been warranted. A paper by McAlister et al.[[16]](#footnote-17) considers how environmental impacts might be considered in these ways. While their ideas are only indicative, they highlight the potential value in considering incremental emissions alongside incremental health benefits and costs to support reimbursement of cost-effective, low-carbon, technologies.



**Importance of international alignment**

Efforts to measure greenhouse gas emissions associated with individual health technology products are aligned with emissions reduction commitments by health technology manufacturers in Australia and internationally. However, it is crucial that these efforts are undertaken in a robust manner that also minimises regulatory burden. To achieve this, international standards for carbon foot printing of health technology products are needed so that manufacturers can undertake a life cycle assessment once for each product using an agreed methodology, which is recognised as valid by health technology assessment bodies around the world. Existing standards for LCA studies are not specific to healthcare (ISO 14040, ISO 14044, and ISO 14067).

In the UK, NICE is working with the British Standards Institution and industry to develop new international standards for carbon foot printing of pharmaceuticals, and has committed to developing an approach for considering environmental impacts as part of approval decisions by 2023–24.[[17]](#footnote-18) Canada’s Drug and Health Technology Agency has committed to adapt methodologies and analyses to assess the environmental footprint of technologies as part of its 2022–2025 strategic plan.[[18]](#footnote-19)

Australia has an opportunity – including through the International HTA Collaboration – to shape the international regulatory environment by contributing to these efforts, and in so doing to ensure that environmental impacts are incorporated into health technology assessment processes in an efficient and burden-minimising way. Failure to seize this opportunity creates a risk that Australia will have international standards imposed on us that we have not had a part in creating.

The Reference Committee therefore considers that: Australia should actively involve itself in efforts to establish new international standards for assessing the environmental impacts of health technology products, that it consider requiring manufacturers to measure and publicly report emissions of products, and that this information is incorporated into approval and reimbursement decisions in alignment with international best practice and developments in comparable countries.

### Options

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| **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.   Note: this is in line with the  [National Health and Climate Strategy 2023](https://www.health.gov.au/our-work/national-health-and-climate-strategy) |

## Mechanisms for continuous review and improvement

### Introduction and current state

Australia’s HTA processes and methodology have been frequently updated since the PBS was introduced in 1993, including major reforms (see[History of HTA reform processes in Australia](#_History_of_HTA)), as well as continuous monitoring and updates to policies, systems and processes.

These processes have led to multiple periodic updates to guidelines. While there have been many of these processes since 1993, there is no formal or systematic approach to reviewing HTA committee guidelines. Major reviews of HTA committee guidelines have occurred at intervals of every 6 to 10 years.

The PBAC Executive currently informally oversees and reviews PBAC policies and processes and has suggested a number of policy and process improvements for optimising HTA processes and decision-making.

The [New Frontier Report](https://www.health.gov.au/resources/publications/inquiry-into-approval-processes-for-new-drugs-and-novel-medical-technologies-in-australia) recommended (recommendation 29) that:

*“The Australian Government amend the National Health Act 1953 (Cth) to formalise the role and powers of the PBAC Executive. The scope of the Executive’s role and powers should be determined by agreement between the Executive and the Department of Health.”*

#### Strategic Agreement

Section 6.1 (Continuous process improvement) of the [2022–2027 Strategic Agreement between the Commonwealth and Medicines Australia](https://www.pbs.gov.au/info/general/medicines-industry-strategic-agreement) provides that:

*“6.1.1 The parties agree that improvement to HTA processes to facilitate earlier patient access to medicines will continue during the Term, informed by:*

1. *the assessment of the Stage 2 PBS Process Improvements*
2. *the outcomes of the HTA policy and methods review and process improvements under this Agreement*
3. *the submissions and evidence given to and the findings and recommendations of the inquiry into approval processes for new drugs and novel medical technologies in Australia being conducted by House of Representatives Standing Committee on Health, Aged Care and Sport (which is ongoing at the time of entry into this Agreement)*
4. *the review of the National Medicines Policy (which is intended to commence in August 2021), and*
5. *key metrics which will be co-designed and jointly agreed by the parties.”*

#### Australian Government process improvements activities

The Australian Government’s HTA improvement activities aim to develop and implement ongoing enhancements to HTA systems/processes to facilitate earlier patient access to medicines, consistent with strategic agreements with the medicines industry. Key activities include the ongoing collection, monitoring and review of PBS Process Improvement metrics, for example ‘time to listing’ and ‘reasons for non-recommendation’ metrics. Process improvement metrics agreed jointly with the medicines industry are published on the [PBS website](http://www.pbs.gov.au/info/general/pbs-process-improvements).   
An independent Portfolio Charging Review was undertaken in 2022 and recommendations included updating the activity-based charging model for PBAC. The Department is conducting a ‘time and motion study’ for PBS listing activities, which is expected to be completed mid-2024 and outcomes reflected in PBS cost-recovery fees from 1 July 2025. Other improvement activities under way include implementing standardised redaction policy for MSAC public summary documents to support improved consistency across the HTA committees and align them with current PBAC processes where possible.

#### PBS Metrics

The Department currently publishes [PBS process improvement](http://www.pbs.gov.au/info/general/pbs-process-improvements) metrics on the PBS website annually. The first metrics report for Stage 1 PBS process improvements implemented on 1 July 2019 was published in August 2020. This includes metrics on Pre-Submission meetings, the Intent to Apply step, Notice of Intent for Pricing process and Pricing Pathways. Following implementation of Stage 2 PBS process improvements on 1 January 2021, a metric report on Stage 2 has also been available, which reports on metrics in relation to the introduction of initial submission categories and resubmission pathways. Further, the [Medicine Status Website](https://www.pbs.gov.au/medicinestatus/home.html) became available in July 2019 to enable consumers to search for and monitor the status of medicines as they progress through the PBS listing process.

The Australian Government has committed to working with Medicines Australia to determine and make publicly available a range of key performance indicators on the time it takes for new medicines to be listed on the PBS, including measures controlled by the medicines industry (such as the time taken to make applications for regulation and reimbursement in Australia and in other countries) and measures controlled by the Commonwealth (such as the time taken to assess applications and resubmission churn).

The Department is continuing to streamline and digitise the processes to support the public funding of health technologies through progressive implementation of, and improvements to, the [Health Products Portal](https://hpp.health.gov.au/). Alongside this work, the Department is developing a metrics framework to measure and report on PBAC assessments.

#### Post-Market Review Program

In 2015, the Government introduced a systematic post-market approach to monitoring medicines in use to inform decision-making at all levels throughout the medicine cycle (from the registration of a medicine right through to its use by consumers). Reviews of cost-effectiveness ensure that the cost of medicines to the PBS appropriately reflects the health outcomes expected and subsequently produced. The [Post-market Review (PMR) program](https://www.pbs.gov.au/info/reviews/subsidised-medicines-reviews) contributes to:

* improved patient safety through a better understanding of adverse events and promoting medicine use in populations for whom they are safe and effective
* ensuring the ongoing viability of the PBS through targeted medicines usage and avoiding preventable wastage or inappropriate prescribing
* a better understanding of medicines utilisation to review intended clinical benefit and inform medicines evaluation processes
* ongoing cost-effectiveness, including through better management of clinical and economic uncertainty, and
* overall improvements to the quality use of medicines and education for patients and prescribers.

These reviews are to ensure the quality use of PBS listed medicines and the ongoing sustainability of the PBS. Reviews can result in changes to listings of individual medicines, groups of medicines, or policy aspects relating to their evaluation or supply. Information on reviews undertaken to date can be accessed on the [PBS website](https://www.pbs.gov.au/info/browse/reviews).

### What we heard from stakeholders

We have heard that stakeholders believe that the current speed of evolution of health technologies and the evidence base and methods for assessment that support their use necessitates more frequent review of guidelines.

### Issues

The Reference committee agrees there needs to be a continuous approach to updating guidelines. We consider that these should be overseen by the executive of advisory committees.

### Options

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| **A program of continuous review and improvement for current HTA policies and methods**  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. 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. |

## Capacity and capability in the HTA systems

### Introduction and current state

There are currently several emerging areas that are challenging the existing HTA workforce in the healthcare sector. These include:

* increasing numbers of health technologies being marketed each year
* greater challenges in assessing them arising from increased complexity and a lower quality evidence base
* greater need for evidence development after health technologies have been funded
* increased expectation from patients, consumers, clinicians and other members of the community to be informed and involved in decision-making.

#### International work-sharing

International work-sharing is one area that can increase the capacity and capability of the HTA workforce.

In May 2021, the Department signed a Memorandum of Understanding with the Singapore Ministry of Health in the field of health cooperation, which will facilitate deeper exchanges of information, knowledge and expertise in health technology assessment.

Further, the Minister for Health and Aged Care, the Hon Mark Butler MP, has recently approved the Department, together with the PBAC and MSAC, to enter into a collaborative arrangement with like-minded HTA agencies in the UK and Canada. Additional Canadian and New Zealand HTA agencies joined the collaboration in September 2023.[[19]](#footnote-20) This arrangement will provide a framework for close and collaborative ways of working to support strategic objectives, which include identifying and progressing opportunities to improve HTA and regulatory collaboration.

The TGA utilises overseas evaluations from comparable jurisdictions through the following:

* comparable overseas regulator (COR) report-based process
* Access Consortium, and
* Project Orbis.

The TGA makes use of assessments from comparable overseas regulators, where possible, in the regulation of prescription medicines. Specific criteria which apply to the COR report-based process are used to help identify opportunities for enhanced international collaboration in the regulation of prescription medicines. The COR report-based process is open to prescription medicine registration applications, where the medicine has received full overseas marketing approval following a *de novo* evaluation. The process can also be used for variations to existing medicines, including extension of indications or new dosage forms and changes to product information documents that would normally require evaluation of clinical data.

The Access Consortium is a coalition of regulatory authorities including the TGA, Health Canada, Health Sciences Authority of Singapore, Swissmedic and the UK's Medicines and Healthcare products Regulatory Agency, that work together to promote greater regulatory collaboration and alignment of regulatory requirements.[[20]](#footnote-21) Its goal is to maximise international cooperation, reduce duplication, and increase each agency's capacity to ensure consumers have timely access to high quality, safe and effective therapeutic products.

The consortium explores opportunities for information and work-sharing in areas including:

* the registration of medicines containing new active substances (including COVID-19 vaccines and therapeutics)
* the registration of generic and biosimilar medicines
* post-market medicine safety information
* development of technical guidelines for industry, and
* alignment of IT systems and architecture.

The TGA has approved a number of new medicines through the Access Consortium’s Access New Active-Substance Work-Sharing Initiative.

Project Orbis, an initiative of the FDA's Oncology Center of Excellence, provides a framework for the collaborative review of promising new cancer treatments among international regulatory partners (Project Orbis partners).[[21]](#footnote-22) It aims to give patients faster access to promising cancer treatments across the globe. The FDA coordinates the selection of applications for Project Orbis in collaboration with the US applicant. Once the potential Project Orbis partners are identified, the FDA will send a proposal to the Project Orbis partners to confirm their interest and availability to participate in a collaborative review process.

### What we heard from stakeholders

Stakeholders expressed a sentiment that there should be support to develop the workforce needed to evaluate health technologies and implement funding decisions.

### Issues

#### Demand for workforce

The Reference Committee appreciates that there are several pressures on HTA capacity and capability. It also acknowledges that many of the options proposed in this paper are complex and require careful consideration including workshopping and analysis prior to implementation, which will require resourcing by all entities involved in HTA in addition to the resource required for implementation and ongoing operation.

#### Local expertise and capacity in health economics and HTA

The [New Frontier report](https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report) identified a significant need for more health economic capacity in Australia and recommended that:

*“the Australian Government develop a labour market and skills strategy to expand the number of health economists in Australia. This could include encouraging training within Australia as well as seeking expertise from overseas”.*

Additionally, specialist expertise in HTA methodology, policy and processes is required to undertake activities associated with HTA evaluations, as well as overall oversight and process improvements.

Sustainable access to health technologies through collaboration between international jurisdictions

We have observed that increasing harmonisation between jurisdictions can improve the efficiency of assessment and application for the payers and sponsors. We have learnt that approaches to form joint-common markets – such as in the Benelux nations (Belgium, the Netherlands and Luxembourg) has worked to increase the incentive for sponsors to market certain health technologies. Given Australia is a small market in the global context, participation in a larger global market may improve its ability to negotiate in relation to purchasing of innovative health technologies.

### Options

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| **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. |

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| 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). |

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| **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:   1. “Work Sharing Initiative” pathway, where concurrent reimbursement submissions are lodged in multiple jurisdictions and dossier modules are work-split amongst participating agencies 2. “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) 3. 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) 4. 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. |

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| **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. |

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# List of Acronyms and Abbreviations

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| **Acronym** | **Full** |
| ACCHSs | Aboriginal and Torres Strait Islander community-controlled health services (also ACCHOs) |
| AT’s | Advanced Therapies (see also ATMP’s and HST’s) |
| AHTA | Adelaide Health Technology Assessment |
| AMR | Antimicrobial resistance |
| ARTG | Australian Register of Therapeutic Goods |
| ATAGI | Australian Technical Advisory Group on Immunisation (Australia) |
| ATMPs | Advanced Therapy Medicinal Products  *also sometimes known as ‘advanced therapies’, ‘highly specialised technologies’, or cell and gene therapies.* |
| AHMAC | Australian Health Ministers’ Advisory Council |
| BPORs | Budget Process Operational Rules |
| CADTH | Canadian Agency for Drugs and Technologies in Health |
| CAR-T | Chimeric antigen receptor T cell therapy (a type of gene modified cell therapy) |
| CCC | Consumer Consultative Committee (Australia) |
| CEA | cost-effectiveness analysis |
| CED | coverage with evidence development |
| CEEU | Consumer Evidence and Engagement Unit (Australia) |
| CMA | cost-minimisation analysis |
| CIRS | Centre for Innovation and Regulatory Science (international) |
| CHERE | Centre for Health Economics Research and Evaluation |
| Consultation Hub | Office of Health Technology Assessment Consultation Hub |
| CUA | cost-utility analysis |
| EMA | European Medicines Agency |
| ESC (PBAC ESC) | PBAC Economic Sub-Committee (PBAC economic sub-committee).  *Note: MSAC also has a sub-committee called the ‘evaluation sub-committee’, which is often also abbreviated to ESC. For the purposes of this document ‘ESC’ refers to the Economic sub-committee, and the MSAC sub-committee will be referred to as MSAC ESC.* |
| FDA | Food and Drug Administration (US) |
| G7 | Group of Seven |
| HATV | high added therapeutic value |
| HST | High cost Highly Specialised Therapy delivered to public hospital inpatients as defined under the NHRA 2020-25 addendum |
| HTA | Health Technology Assessment |
| HTA Review | Health Technology Assessment Policy and Methods Review |
| HUCN | High Unmet Clinical Need |
| ICER | Incremental Cost Effectiveness Ratio  *Note: the Institute for Clinical and Economic Review also abbreviates its name to ICER, so will be referred to in full in this document).* |
| the Inquiry | Standing Committee on Health, Aged Care and Sport (Standing Committee) Inquiry into approval processes for new drugs and novel medical technologies in Australia |
| IHSI | International Horizon Scanning Initiative |
| LSDP | Life Saving Drugs Program |
| MAP | Managed Access Program  also sometimes known as Manages Entry Programs…  *Note: not to be confused with ‘Medicines Access Programs’.* |
| MBS | Medicare Benefits Schedule (Australia) |
| MCDA | multiple criteria decision analyses |
| MEAs | managed entry agreements  (includes Managed Access Programs (MAPs), risk-share arrangements (RSAs)). |
| MI-CRE | Centre of Research Excellence in Medicines Intelligence |
| MSAC | Medical Services Advisory Committee (Australia) |
| MSAC ESC | MSAC evaluation sub-committee (Australia)  *Note: not the same as ESC, economic sub-committee)* |
| NACCHO | National Aboriginal Community Controlled Health Organisation |
| NBA | National Blood Arrangements (Australia) |
| [NHRA](https://www.health.gov.au/our-work/2020-25-national-health-reform-agreement-nhra) | National Health Reform Agreement |
| NICE | National Institute for Health and Care Excellence (UK) |
| NITAG | National Immunisation Technical Advisory Group |
| NIP | National Immunisation Program (Australia) |
| NIHRIO | National Institute for Health and Care Research Innovation Observatory (UK) |
| NMP | [National Medicines Policy](https://www.health.gov.au/resources/publications/national-medicines-policy?language=en) |
| The New Frontier Report | The New Frontier - Delivering better health for all Australians, Report from the Inquiry (see above) |
| NHS | National Health Service (UK) |
| OECD | Organisation for Economic Co-operation and Development |
| PBAC | Pharmaceutical Benefits Advisory Committee (Australia) |
| PICO | Population, Intervention, Comparator, and Outcome |
| PBS | Pharmaceutical Benefits Scheme (Australia) |
| PhRMA | Pharmaceutical Research and Manufacturers of America |
| PO | pricing offer |
| PREM | Patient Reported Experience Measure (PREM) |
| PROM | Patient Reported Outcome Measure |
| QALY | quality adjusted life year |
| RCTs | randomised controlled trials |
| RPBS | Repatriation Pharmaceutical Benefits Scheme (Australia) |
| RWD | Real-World Data |
| RWE | Real-World Evidence |
| SLE | systemic lupus erythematosus |
| SOC | standard of care |
| STEDI | Spectrum, Transmission, Enablement, Diversity, Insurance Value |
| TGA | Therapeutic Goods Administration (Australia) |
| TIS | tisagenlecleucel (a type of CAR-T therapy) |
| UK | United Kingdom |
| US | United States |
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