Accelerating Access to the Best Medicines for Australians Now and into the Future

A review of Australia's health technology assessment policies and methods for the Australian Government

Cover artwork

These four designs represent culture, family, body and mind.

Family – Family holds a great importance in Aboriginal culture. It extends beyond the immediate family to include extended mob and community members forming a stronger kinship system to establish responsibilities and connection.

Community – Community provides a support system for our mob to ensure we are connected and have access to services we require. Within our communities they provide a network where traditions, cultural practices and knowledge is shared and passed down to our younger generation.

Body – Ensuring our Aboriginal people have a healthy body, which supports active participation in community life and the transmission of cultural practices and knowledge. Promoting and maintaining a healthy body for Aboriginal people is not just for individual wellbeing but it is also deeply connected to cultural care, social cohesion and historical resilience.

Mind – Engaging in cultural practices, language, art, dancing and hunting fosters a strong sense of identity and belonging, which are crucial for mental wellbeing.

Kaya (Hello), my name is Jacinta Anderson and I am a proud Noongar Yorga with family connections to the Mineng area in the Great Southern, the Yuet area in the Wheatbelt region and Whadjuk area.

Within my job role as a mentor, we used art as a way for the girls to connect with culture, storytelling and to build positive relationships. Creating art with the girls inspired me to get more creative and to start creating my own art. I first started painting on wooden serving boards, which led to a few commission pieces for family and friends to now creating artwork for companies, creating digital art, and running art workshops. I love expressing my culture throughout my art, especially using Aboriginal symbols.

I create commissioned pieces, both acrylic paint on a canvas and digital.

Instagram page – artby_cinta

Health Technology Assessment
Policy and Methods Review

Dear Minister

I am pleased to provide you with the final report of the Health Technology Assessment (HTA) Policy and Methods Review.

Australian citizens were placed at the centre of this comprehensive, evidence-based HTA Review. The HTA system must serve Australians' interests. The Review Reference Committee has therefore sought to ensure that the outcomes directly align with the interests and wellbeing of those in our community.

We commissioned academic centres to undertake a rigorous comparative analysis of international HTA systems. We learnt from this policy and methods analysis, and we found a world-class HTA system in Australia—albeit one that can be improved.

The Review was a collaborative effort, engaging a diverse range of individuals and organisations – including patient groups, industry representatives, clinicians and government bodies. This inclusive approach not only captured the diverse needs and perspectives of those involved in healthcare delivery and decision-making but also fostered transparency, accountability and stakeholder buy-in, thereby enhancing the relevance and effectiveness of the Review's recommendations. I extend my heartfelt gratitude to those organisations and people who engaged with us and shared their expertise, insights and experiences.

The Review has identified areas of consensus by incorporating the needs and preferences of different interests. However, in a system that affects the lives of thousands of Australians, includes hundreds of stakeholder groups, and involves billions of dollars, there will also be areas of tension. I believe that our approach has delivered balanced and informed recommendations that account for the complex and multifaceted nature of health care.

By its nature, a policy and methods review is technical, and some of the recommendations in this Review relate to complex systems issues. However, implementing them as a package over the short, medium and long terms – with the shared commitment, partnership, responsibility and stewardship of all stakeholders – will provide direct benefits for Australian citizens, consumers and patients. Following are examples of the benefits this will create.

Substantial reduction in medicine approval times

The Reference Committee believes, subject to the acceptance of the Review's recommended reforms, that:

- Positive HTA recommendations should be achieved within two submissions for products demonstrating superiority.
- If submissions to the Therapeutic Goods Administration (TGA) and the Pharmaceutical Benefits Advisory Committee (PBAC) for product registration and listing were considered in parallel using an effective process, 90% of products demonstrating superiority could be PBS-listed within 6 months of TGA registration. This is a substantial decrease compared to the current median time frame of 22 months.
- Vaccines and life-saving medicines for people with ultra-rare diseases could be subsidised faster, in 18–22 weeks and 4 weeks, respectively, subject to acceptance and implementation of the Review's recommended reforms.

The Reference Committee considers that such a significant reduction in medicines approval times is the jointly owned responsibility of partner stakeholders – and supports the government and industry goals of minimising the time needed to complete HTAs and finalise commercial agreements for important health technologies.

More timely and equitable access to new and emerging treatments

The Reference Committee believes several proposed reforms will provide more timely and equitable access to new and emerging treatments while managing the uncertainty and risk that often contribute to delays in offering patients subsidised access to health technology.

One example is the establishment of a bridging fund for eligible therapies of high added therapeutic value that address high unmet clinical need.

Better involvement of those impacted by HTAs

The Review makes multiple recommendations to improve transparency and the involvement of patients, consumers, health professionals and others who are impacted by HTAs, and whose participation and input greatly benefit the assessment process and resulting outcomes.

I want to thank my fellow Reference Committee members for their expertise, tireless work and collaborative engagement and thank you for the opportunity to lead this Review into such an important policy area. The Review benefited from their exceptional range of clinical, academic, health delivery, policy and industry skills. Their direct and longstanding leadership roles in health technology assessment ensured we remained patient- and consumer-centric.

Finally, I would like to thank the Review Secretariat for their invaluable contributions to this Review. Their exceptional skills, expertise, unwavering integrity, and commitment to the healthcare needs of citizens have been fundamental to our success. This Review could not have been accomplished without their support.

Adjunct Professor Debora Picone AO
Independent Chair
HTA Policy and Methods Review

31 May 2024

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Preamble

Acknowledgement of Country

We acknowledge the Traditional Owners and Custodians of Country across this nation on whose lands we all work, play and live. We acknowledge their ongoing connection to land, waters and community. We pay our respect to Elders past, present and emerging.

Health Technology Assessment Policy and Methods Review Reference Committee

Member	Role on the Reference Committee
Adjunct Professor Debora Picone AO	Independent Chair
Dr Dawn Casey PSM	Patient representative
Ann Single	Patient representative
Professor Andrew Wilson AO	Chair of the Pharmaceutical Benefits Advisory Committee
Professor Andrew Roberts AM	Clinical/scientific representative
Elizabeth de Somer	Member nominated by Medicines Australia
Adjunct Professor Adriana Platona PSM	Member nominated by the Australian Government

Acknowledgements

The Health Technology Assessment Policy and Methods Review Reference Committee would like to acknowledge the significant contributions to the Review by the members of the Review Secretariat:

Malanie Banney, Daniel Chaston, Jeff Chau, Abby Ching, Eliana Della Flora, Lauren Kucka, Christopher Lee and Yun Zhong.

Additionally, the Reference Committee would like to thank the many staff within the Technology Assessment and Access Division and across the Department of Health and Aged Care for their contributions.

The Reference Committee would also like to thank the dedicated stakeholders: a diverse range of groups and individuals who meaningfully and constructively contributed to the Review.

Executive summary

Australia's health system and health technology assessment processes

The foundation of Australia's world-class universal health system is Medicare, which guarantees all Australians have access to a wide range of health and hospital services at low or no cost. Health care is delivered through subsidised access to a wide range of health services and health technologies, and multiple different subsidy and funding programs. These include the Medicare Benefits Schedule (MBS), the Pharmaceutical Benefits Scheme (PBS), the National Immunisation Program (NIP) and the Life Saving Drugs Program (LSDP). Committing taxpayer funds to these programs is part of the Australian Government's broader goal of improving health and living standards for all Australians.

Health technology assessment (HTA) is the process that supports decision-making in funding health services and technologies (such as medicines, vaccines and medical devices) in the health system. Australia's HTA processes were a focus of the House of Representatives Standing Committee for Health, Aged Care and Sport's 'Inquiry into approval processes for new drugs and novel medical technologies in Australia' (The New Frontier inquiry).[1] Like this Review, The New Frontier inquiry found there is work to do to improve health technology assessment policy and methods in Australia to be more transparent, engaging, equitable, and reducing the time for patients to access subsidised medicines, especially when their need is high and urgent.

The main policy framework that guides access for health technologies is Australia's National Medicines Policy (NMP). This 'identifies and brings together all partners around a common aim and shared responsibility for policy stewardship. Its vision is to achieve the world's best health, social and economic outcomes for all Australians through a highly supportive medicines policy'.[2]

Australia's NMP has four central pillars that seek to ensure:

- equitable, timely, safe, and reliable access to medicines and medicine-related services, at a cost that individuals and the community can afford
- medicines meet the required standards of quality, safety and efficacy
- quality use of medicines and medicines safety

[1] Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier - Delivering better health for all Australians* inquiry.

[2] Department of Health and Aged Care (DHAC) (2022) *National Medicines Policy 2022*.

- a collaborative, innovative, and sustainable medicines industry, and research sectors with the capability, capacity and expertise to respond to current and future health needs.[3]

For many decades, HTAs have played a central role in meeting the objectives of these pillars. Decision-makers use HTAs to reach determinations about which health technologies to subsidise to support safe, effective healthcare improvements for Australians. HTA helps decision-makers understand how well a health technology (a medicine, technology or service) is likely to perform compared to the existing standard of care for patients, as well as the cost to the taxpayer of any improvements to health outcomes the technology provides.

The Australian Government relies on advice from independent expert committees comprising medical practitioners, health professionals, health economists, other experts and consumer representatives. Members are appointed to be the pre-eminent source of advice on decisions related to subsidising health technologies (including for whom and at what cost) and they use HTAs to inform that advice.

Therefore, Australia's processes, including HTAs, that support decisions about medicines access, pricing and funding enable Australians to receive affordable health care, while providing assurance to the Australian Government and citizens that decisions to spend more on health technologies are transparent and accountable, and will result in better healthcare access and improved health outcomes.

The Review

The system that provides rigorous and defensible HTA advice to the Australian Government about which health technologies should be funded, for whom, and under what circumstances, needs to be able to respond to the rapid pace and advances in medical technologies. The opportunities presented by advances in medical science to benefit the health of Australians are significant. The health technologies emerging today, and those that are likely to emerge in the near future, are also increasingly diverse, complex and expensive. Hence, the role of HTAs in enabling governments to deliver equitable, universal and the best possible health care is becoming even more important.

It is also important that citizens are confident that the HTA arrangements are person-centred, rigorous and effective, and are set up for the future. Successive strategic agreements between the Australian Government and industry have led to numerous incremental improvements in HTA processes and methods. This includes the

[3] DHAC (2022) *National Medicines Policy 2022*.

2022-2027 Strategic Agreement between the Commonwealth Government and Medicines Australia,[4] which reflects a common interest in:

- delivering greater longer-term certainty for patients, the pharmaceutical industry and the Commonwealth through a predictable PBS
- timely access to new medicines
- strengthening the Australian medicines ecosystem by:
  - encouraging companies to continue to bring to Australia innovative medicines that deliver better health outcomes for patients
  - building partnerships with Australian researchers
  - encouraging companies to invest in vaccines, new technologies, local clinical trials, research and development, manufacturing and Australian jobs
  - maintaining Australia's position as a global priority for organisations launching new and innovative medical treatments.

The Review has presented a significant and timely opportunity to examine Australia's approach to HTA. To understand the issues – and opportunities for improvement – the Review's objectives under its Terms of Reference included working with stakeholders to identify features of HTA policy and methods 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 Review also provides an opportunity to examine how HTA policy, methods and processes are influenced by the NMP. This includes the Review's observations on achieving the vision and aim of the NMP through partnerships, which are:

- All partners must be engaged in a collaborative and cooperative manner to achieve the best health, social and economic outcomes for all Australians.
- Each partner has a role in progressing the NMP by demonstrating respect for, and recognising the expertise and contribution of, other partners, with some partners having additional responsibilities for achieving the NMP's objectives.
- The success of the NMP relies on shared decision-making, strategic partnerships and the involvement of people with lived experience in the co-design, development, implementation and evaluation of related policies, strategies, programs and initiatives.[5]

Extensive consultation with consumers, clinicians and industry informed the Review's recommendations. The full consultation process included:

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[4] DHAC (2022) *Strategic Agreement between the Commonwealth of Australia and Medicines Australia 2022-2027*. Pharmaceutical Benefits Scheme (PBS).

[5] DHAC (2022) *National Medicines Policy 2022*.

- commissioning of seven external expert papers and two departmental research papers[6]
- holding two public consultations (April to June 2023, and January to February 2024)
- conducting deep dives with stakeholders (26 sessions with 116 participants).

Objectives of the Review's recommendations

A multifaceted approach will be required to improve the HTA arrangements, in part due to the complexity of Australia's health system. The Review recommends reforms of HTA policy and methods that provide stakeholders and decision-makers with tools and processes to:

1. address inequities in access
2. improve timely access to medicines
3. improve engagement
4. invest in HTA capability to make it adaptable and futureproof.

The package of reforms is comprehensive, and range from horizon scanning, planning and system readiness to bringing important medicines and vaccines to Australia, and evidence assessment, implementation and review. The central tenet of the Review, reflected through extensive engagement with the Australian community, is the imperative to improve timely access to affordable medicines and vaccines to meet the needs of all Australians, in particular First Nations people.

Addressing inequities in access

First Nations people

The Review recommends reforming HTA mechanisms to appropriately engage First Nations people in decision-making processes so that their priorities and perspectives are integrated across the HTA continuum. Despite a range of measures aimed at supporting First Nations people's access to medicines, it was previously estimated the average expenditure on the PBS and Repatriation Pharmaceutical Benefits Scheme (RPBS) is $167 per person for First Nations people compared to $427 per person for non-Indigenous Australians (based on 2015-16 data).[7] These figures bring into sharp focus the issues First Nations people face in gaining equitable access to medicines.

Stakeholders also reported that many medicines that are integral to the health and wellbeing of First Nations people are not listed on the PBS. Addressing these inequities requires all partners to commit to playing their part. The Review therefore recommends

[6] DHAC (2023) *Health Technology Assessment Policy and Methods Review – Research and analysis papers*.

[7] Australian Institute of Health and Welfare (2020) *National Indigenous Australians Agency, Aboriginal and Torres Strait Islander Health Performance Framework: 3.15 Access to prescription medicines*.

HTA committees and supporting processes address these inequities through meaningful engagement and priority-setting with First Nations people.

Paediatrics

Access to PBS-listed medicines for paediatric patients including children, adolescents and teenagers needs to be increased. There is a lack of medicines for treating paediatric indications, which is mostly attributable to challenges in generating the clinical data on use of health technologies by paediatric patients needed for regulatory approval, and consequently reimbursement. It is noted that the return on investment for paediatric indications often does not offset the costs associated with the complexities of conducting clinical trials for paediatric patients.

The Review noted instances where the Pharmaceutical Benefits Advisory Committee (PBAC) has omitted age from PBS restrictions, allowing broader access for children than that approved by the Therapeutic Goods Administration (TGA). However, age-agnostic PBS listings have not been applied in a systematic manner, which has likely contributed to unequal access for paediatric patients across the PBS.

A lack of clinical trials in paediatric populations is a fundamental barrier to improving access for paediatric patients. However, the policies and regulations guiding clinical trial development are beyond the scope of the Review. While the TGA's processes are also beyond the Review's scope, increased collaboration between regulatory and HTA bodies in addressing some of the challenges with paediatric trial data could facilitate timely regulatory approval through to HTA. The Review's recommendations are intended to improve access to PBS-listed medicines for paediatric patients and facilitate regulatory approval to enable reimbursement of medicines for paediatric indications.

Reducing wait times for access to affordable medications

In 2021 and 2022, submissions for new drugs demonstrating superiority required on average more than two considerations by the PBAC (range 2–4) and only around 50% were listed on the PBS within 22 months of Australian Register of Therapeutic Goods (ARTG) registration (i.e. TGA approval). There are significant opportunities to improve HTA arrangements to reduce wait times for access to medicines through improvements to different parts of the HTA systems and processes.

Pathways

Australia has several pathways for assessing health technologies offered by organisations seeking Australian Government funding. Each distinct funding and assessment pathway incorporates a range of processes and decision points and involves several decision-makers and stakeholders.

The Review's findings underscore the world-class quality of Australia's HTA system and processes and the strength of Australia's medicines funding systems. However, we can strive for improvement. The Review sought to identify opportunities to improve the current system and processes, to provide timely access to medicines and sustainably meet the needs of Australians into the future.

Government, industry and all stakeholders share responsibility for working together to ensure the health system continues to successfully deliver better health, wellbeing and value for all Australians.

The Review's findings and recommendations could significantly improve timely access to important – and transformative – health technologies. This will be achieved through reducing duplication and increasing consistency between the different HTA pathways, and ensuring the time and effort of the entire system is directed where it will be most beneficial.

The Review recommends mechanisms to improve equitable and timely access across the entire HTA ecosystem. This includes the time to gain subsidised access to vaccines on the NIP, life-saving drugs for people with ultra-rare diseases, and highly specialised therapies (HSTs) delivered through the 2020–25 Addendum to the National Health Reform Agreement (NHRA).[8] It also includes the time for therapies proposed for listing on the PBS and co-dependent technologies.

If its recommendations are accepted by Government, the Review believes it will be possible to achieve positive HTA recommendations after one submission, or no more than two submissions, for products demonstrating superiority. It also believes 90% of these products could be listed on the PBS within 6 months of ARTG registration when parallel processing is used effectively, or within 12 months if it is not used. Having jointly owned performance targets would support the shared government and industry goal of minimising the time to complete HTAs and commercial agreements for important health technologies that are superior to existing therapies.

Bridging fund

The Review recommends establishing a bridging fund to reduce waiting times for health technologies with high added therapeutic value that target areas of high unmet clinical need (HUCN).

Stakeholder feedback from patients, industry members, clinician groups and healthcare payers (i.e. governments) consistently emphasised the importance of alternative approaches to managing clinical, economic and/or budget impact uncertainties identified during an HTA evaluation.

[8] Australian Government (2020) *2020–25 Addendum to the National Health Reform Agreement*.

Alternative approaches used internationally at a broad health systems level include special funding (bridging) programs that:

- balance the need to manage the mix of uncertainty and risk identified during the HTA process at a more general health expenditure level (by using funding that is distinct from standard healthcare expenditure arrangements)
- provide an avenue for health technologies of high clinical significance to reach patients earlier than standard access approaches (to improve health equity outcomes) while identified uncertainties are resolved (for example as part of further evaluation, stakeholder negotiation or data collection).

In recommending a bridging fund, the Review noted from international experiences that large financial outlays are required to set up such a fund. These arrangements succeed when stakeholders negotiate and participate in them in good faith, and deliver on agreed responsibilities and obligations in a timely and efficient manner (including in cases where it is necessary to withdraw bridging funding and transition away from using a given health technology).

Antimicrobial resistance

The Review recommends incentives for companies to supply and market antimicrobials in Australia.

The rise of antimicrobial resistance has significant effects on disease burden and poses a threat to the financial sustainability of global health systems and the broader economy. However, due to a low return on investment through existing reimbursement methods, the market is failing to incentivise the development of new antimicrobials.

There are opportunities across HTA processes and policies to support greater investment in developing new antimicrobials, including by removing barriers in the market authorisation, evaluation and funding of antimicrobials. It is noted that work has begun on scoping potential funding mechanisms. However, action is required to reduce disincentives for organisations seeking funding to develop antimicrobials.

Improving transparency and engagement

Engagement framework

Effective stakeholder engagement is fundamental to achieving the goals of the NMP[9] and good HTA processes and methods. Having an engagement framework for the entire HTA continuum would help fully realise the potential value of stakeholder involvement to support HTA committee decisions. The Review acknowledges that there is a complementary an Enhanced Consumer Engagement Process currently under way.

[9] Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier - Delivering better health for all Australians* inquiry.

This work was initiated as an element of the *2022–2027 Strategic Agreement between the Commonwealth and Medicines Australia*,[10] and is being undertaken via a consumer-led co-design process informed by consumers, representatives of the Department of Health and Aged Care (the Department), industry representatives, and other stakeholders.

Improving communication and transparency

Multiple solutions are required to improve communication and transparency, to optimise how patients, consumers, health professionals and other stakeholders engage across all HTA processes.

During consultations, the Review heard that stakeholders often cannot find the information they need relating to HTA policies, systems or submissions. Further, stakeholders are not satisfied with existing plain language explanations of HTA pathways and guidelines. While much information is available across the different HTA websites, it is difficult to navigate or understand as it is presented in technical language, is ambiguous, or deals with concepts that are not well explained. The Review found the lack of clear and accessible information results in many misconceptions about the HTA systems, processes and policies further demonstrating the need for plain language communications to enhance clarity and understanding.

The Review recommends a range of initiatives across the HTA continuum to better engage with those impacted by HTA decisions and to improve transparency. These include:

- providing plain language summaries to facilitate greater stakeholder and consumer understanding, engagement and input
- improving transparency about how stakeholder perspectives have been taken into account in decision-making
- clearly communicating HTA committee decisions and rationale, including developing an explicit qualitative values framework
- increasing accessibility and visibility of key information (including through channels such as the HTA website) about a health technology at different stages of its lifecycle to support transparency.

Population, Intervention, Comparator, and Outcome framework

Determining an HTA Population, Intervention, Comparator, and Outcome (PICO) framework is a key factor in making consumers' access to health technologies more equitable. By defining the scope of analysis for an HTA, a PICO framework guides HTA

[10] DHAC (2022) *Strategic Agreement between the Commonwealth of Australia and Medicines Australia 2022-2027*.

advisory committees' decisions about which patients a health technology will be recommended for.

Patients and clinicians have minimal opportunities to participate when sponsors of HTA submissions formulate the PICO framework. Greater access to key information about a health technology and broader dialogue on development of PICO frameworks in HTA submissions could make the process more patient-centred and assist in the appraisal of health technologies in certain circumstances. This could help to reduce inequitable access arising from the exclusion of populations that could potentially benefit from therapy (such as paediatric populations).

Horizon scanning

The Review recommends establishing a horizon scanning function.

It found that systematic collection, analysis and sharing of information on health technologies in the research pipeline and entering clinical practice (i.e. horizon scanning) is essential to support an effective and efficient HTA program. Horizon scanning allows stakeholders to be better informed about the benefits, risks and operational implications of new health technologies. It also prompts earlier conversations about health systems planning to support the introduction of new health technologies. The Review also found that Australia does not perform any formal horizon scanning activities at the national level.

In making its recommendations supporting the development of a horizon scanning function, the Review emphasises that this function is highly resource-intensive in terms of financial costs, time and effort. It also requires stakeholders to clearly define and plan the scope, purpose and utility of the activities to ensure success in Australia.

Investing in HTA capability to be adaptable and futureproof

Real-world evidence

The Review found that a key component supporting the operation of the HTA system should include a coordinated and standardised approach to collecting real-world data (RWD) and real-world evidence (RWE) to understand how health technologies are being used and are performing in the real world. Where timely, high-quality RWE has been used to enhance and complement available randomised controlled trial (RCT) evidence, it can be influential in reducing the uncertainty in decision-making and increasing timely access to therapies through the ability to monitor health outcomes post listing.

Although some jurisdictions collect RWD and generate RWE for some diseases and/or health technologies, their approaches are often not coordinated or standardised. The

data may not be configured to answer questions about clinical effectiveness and cost-effectiveness, and there is often considerable duplication of effort. The Review found that gaining timely access to sufficient, relevant, quality data can be challenging due to limited or disjointed resourcing and infrastructure, legislative and administrative issues, privacy and security concerns, and confusion among some stakeholders about how RWE is used in HTA.

The Review recommends establishing structures, policies and methods that support the timely collection and use of relevant RWD and RWE, including:

- a comprehensive, multi-stakeholder data strategy, with strategic oversight
- national, coordinated data infrastructure, standards and guidance.

Capability – Workforce and general resourcing of reform implementation

Effective implementation of the Review's recommendations will require additional and adequate resourcing.

The Review found that while Australia's HTA processes serve the nation well, the increasing complexity, rapidity and volume of new technologies to be assessed, combined with growing consumer and industry expectations, are placing increased pressure on Australia's HTA system and its comparatively small workforce.

The number of submissions to the PBAC, and the number of new and expanded listings on the PBS, have grown steadily each year over the past 10 years. The emergence of personalised medicines, such as gene and gene-modified cell therapies, means that the number and complexity of applications needing HTA assessment will continue to grow. For example, Australia's HTA capability and processes must prepare for, and be able to respond to, the volume of gene-modified cell therapies in the pipeline. The following numbers indicate the scale of the HTA tasks ahead. As of January 2024, 7 cell and ribonucleic acid (RNA) therapies were registered in Australia; 46 were registered in other comparable jurisdictions; and 3,951 were in clinical development.

The reforms recommended by the Review will place further demands on the HTA system. For example, improving communication and engagement processes, enabling proactive activities such as horizon scanning, streamlining funding and approval pathways, and establishing and operating a bridging fund all require additional resources and investment in the workforce and system capabilities. The Review emphasises that these recommendations cannot be delivered effectively with the current departmental appropriations.

Conclusion

The Review provided a significant and timely opportunity to examine Australia's approach to HTA: to determine what is working effectively, how to improve the settings

and how to futureproof the system to best meet the opportunities and challenges of the future.

The Reference Committee members for the Review recognise that implementing such a multifaceted program of reforms will be complex and costly. It also needs to occur in sync with other reforms arising from The New Frontier inquiry,[1] where the Australian Government has made a commitment to advance these reforms. HTA policy and methods will need to continuously improve to ensure they remain relevant and meet community expectations while assuring the Government and citizens that decisions to spend more on health technologies will result in even better healthcare access and improved health outcomes.

Summary of recommendations

The following summarises the Review's key recommendations for the Australian Government to action, in collaboration with relevant stakeholders. The full, detailed recommendations are contained within the relevant chapters.

CHAPTER 3: PROVIDING MORE EQUITABLE ACCESS TO UNDER-REPRESENTED PATIENT GROUPS

Recommendation 1. Creating a more equitable system for First Nations people

Reduce health inequity for First Nations people by:

- establishing a First Nations Advisory Committee to advise the Pharmaceutical Benefits Advisory Committee and the Medical Services Advisory Committee on matters for First Nations people such as priority indications for people with high unmet clinical need
- including First Nations representative/s on the Pharmaceutical Benefits Advisory Committee with relevant expertise and experience to provide information relating to the of health and equity impacts of submissions on First Nations people
- requiring sponsors' submissions to include consideration of the impact on health outcomes for First Nations people.

Recommendation 2. Providing equitable access to medicines for paediatric patients

Adopt an age agnostic approach for new listings on the Pharmaceutical Benefits Scheme unless special circumstances necessitate restricting access.

Establish a working party led by representatives of the Pharmaceutical Benefits Advisory Committee and the Therapeutic Goods Administration to develop guidance on extending the use of Therapeutic Goods Administration registered therapies to paediatric populations.

CHAPTER 4: STREAMLINED PATHWAYS FOR MORE TIMELY ACCESS

Recommendation 3. Overarching recommendations for all HTA funding and assessment processes and pathways

Reform health technology funding and assessment processes and pathways to align them with the following overarching principles.

Streamlined and simple

Streamline and simplify health technology funding and assessment processes by removing unnecessary complexity and redundancy.

Proportionate and fit-for-purpose

Restructure and develop health technology funding and assessment processes, including the level of evaluation, to be fit-for-purpose and proportionate to the level of risk, complexity and potential benefit of a therapy.

Unified and consistent

Create consistency and clarity for all health technology submissions for Australian Government funding by developing a unified HTA pathway and committee approach in stages, starting with better aligning the HTA pathways and removing duplication.

Recommendation 4. Unified HTA pathway and committee approach for all Australian Government funding of health technologies

Working in stages, align health technology funding and assessment pathways, processes and committees, and remove duplication, to create a single unified HTA pathway and committee approach. Stages should include developing a 'single front door' and triaging mechanism, and expanding the advisory role of the Pharmaceutical Benefits Advisory Committee beyond the Pharmaceutical Benefits Scheme, ultimately leading to a single unified committee approach.

Recommendation 5. Triaging submissions

Develop processes that enable triaging of submissions to determine the appropriate evaluation and appraisal mechanisms.

Recommendation 6. Expanding the advisory role of the Pharmaceutical Benefits Advisory Committee beyond the Pharmaceutical Benefits Scheme

As a stage in the development of the unified HTA pathway, expand the advisory role of the Pharmaceutical Benefits Advisory Committee to enable it to make HTA recommendations to the Minister for Health and Aged Care for a broader range of health technologies across different funding and subsidy programs.

Recommendation 7. Streamlined pathway for submissions applying for listing on the Pharmaceutical Benefits Scheme using cost-minimisation analysis

Develop a streamlined assessment pathway for submissions using cost-minimisation analysis.

Recommendation 8. Therapies with high added therapeutic value in areas of unmet clinical need applying for Pharmaceutical Benefits Scheme listing

Enhance or replace the current early resolution and / or facilitated resolution pathway with a more flexible pathway to provide additional support for the submissions of therapies with high added therapeutic value in areas of unmet clinical need. This should be supported by case management for submissions, and allowing the Pharmaceutical Benefits Advisory Committee to provide its likely advice to sponsors earlier before the receipt of the Therapeutic Goods Administration delegate's overview.

Comment by Ms Elizabeth de Somer, Member Nominated by Medicines Australia: 'The industry recognises the need to ensure no perverse incentives are introduced into these

pathways and recommends establishing independent dispute resolution and commercial negotiation processes.'

Recommendation 9. Therapies with added therapeutic value

After a trial period and review, extend the mechanisms in Recommendation 8 from therapies with high added therapeutic value in areas of unmet clinical need applying for Pharmaceutical Benefits Scheme listing to all therapies claiming clinical benefit over existing alternatives.

Recommendation 10. Alternative modelling and analysis types for disease areas

In consultation with industry and other relevant stakeholders, investigate the feasibility and a potential place for alternative types of analysis and modelling for disease areas.

Recommendation 11. Proportionate appraisal pathway to align Australian Technical Advisory Group on Immunisation assessments with the level of risk and complexity of the product

Restructure the current pathway for listing a vaccine on the National Immunisation Program to better align the Pharmaceutical Benefits Advisory Committee and Australian Technical Advisory Group on Immunisation processes, and create assessment processes that are proportional to the level of complexity, risk and benefit of a submission.

Recommendation 12. Proactive vaccine assessment pathway

Develop a process to enable proactive consideration of how new products or potential changes to the vaccine program could impact disease burden. This process should be developed in collaboration with the Australian Technical Advisory Group on Immunisation and other relevant stakeholders and include conducting independent modelling, where appropriate.

Recommendation 13. Improved processes, accountability and timeliness for highly specialised therapies and other therapies co-funded by the Australian and state and territory governments

Encourage and provide support for expediting the development and implementation of a nationally cohesive approach to HTA as outlined in Schedule C of the 2020–25 Addendum to the National Health Reform Agreement.[11]

Encourage and provide support for expediting the development of a national HTA framework, including processes for HTA to inform advice on implementation, investment and disinvestment opportunities at Commonwealth and state and territory levels.

Work with state and territory governments and industry to establish processes for ensuring high-cost highly specialised therapies are accessible to all eligible patients within 6 months of reaching an in-principal pricing agreement.

[11] Australian Government (2020) *2020–25 Addendum to the National Health Reform Agreement*.

Develop a framework for systematic input, consultation and work sharing by state and territory governments across the health technology lifecycle.

Recommendation 14. Improving time to access life-saving drugs for patients with ultra-rare diseases (Life Saving Drugs Program)

Develop a statement of rationale for the Life Saving Drugs Program, outlining the principles underpinning the program and eligibility criteria.

Make necessary process and policy reforms to enable the Pharmaceutical Benefits Advisory Committee to become the sole HTA committee that assesses and recommends funding of health technologies for ultra-rare diseases.

Recommendation 15. Jointly owned performance targets

Get the Australian Government and industry to each reaffirm their commitment to good faith negotiations aimed at minimising the time to complete an HTA and commercial agreements for products claimed to be superior to existing therapies. In addition, they should negotiate reciprocal commitments for these elements in any agreement, and compile and publish performance metrics annually.

Comment by Ms Elizabeth de Somer, Member Nominated by Medicines Australia: 'The industry supports mutually agreed targets that reduce delays in patient access and recommends that a time frame for PBS listing within 60 days of ARTG registration for all submissions should be a future target.'

CHAPTER 5: POLICIES, METHODS AND PROCESSES SUPPORTING THE TRANSLATION OF HTA RECOMMENDATIONS INTO PATIENT ACCESS

Recommendation 16. Addressing the implications of high-cost/high-impact health technologies

Design a framework that supports using different contract and funding mechanisms to subsidise health technologies, in addition to the standard 'price per unit' approach.

Recommendation 17. Pricing offer framework

Publish (after appropriate consultation and development) a regularly updated post-HTA pricing, negotiation and listing policy framework that provides stakeholders with clarity and visibility about matters relevant to translating a positive HTA recommendation into subsidised access for patients.

Recommendation 18. Updated post-review framework

Build on existing health technology review and evaluation arrangements to support regular and periodic examination of the performance, utilisation, displacement, and clinical place of health technologies and include activities supporting reviews throughout a health technology's post-listing utilisation lifecycle.

Recommendation 19. Managed entry agreements

Revise the policy and guidance framework of managed entry agreements, to provide more flexibility for sponsors and the Commonwealth to address identified uncertainties while better supporting timely access to health technologies for patients.

Recommendation 20. Bridging funding program

Establish a bridging fund to facilitate earlier, temporary subsidised access to eligible therapies of high added therapeutic value that address high unmet clinical need for patients.

Recommendation 21. Approaches to incentivise development of health technologies that address antimicrobial resistance

Implement measures to incentivise the development of antimicrobials including:

- exempting them from HTA fee requirements
- developing a framework to inform changes to HTA policy and methods for antimicrobials
- designing a flexible reimbursement policy for antimicrobials, including examining and testing multiple payment and incentive models including establishing a subscription model to fund novel antimicrobials in the short term.

CHAPTER 6: TRANSPARENCY AND STAKEHOLDER INVOLVEMENT

Recommendation 22. Publishing plain language summaries

Provide plain language summaries of Pharmaceutical Benefits Advisory Committee submissions (developed by each submission's sponsor and the Department of Health and Aged Care in collaboration) at the time of publishing the Pharmaceutical Benefits Advisory Committee agenda so that consumers have enough information to participate in the HTA and understand the expected benefit of the therapy and the proposed population, without ambiguity.

Recommendation 23. Improving the HTA website including the development of a dashboard

Enhance the HTA website by improving navigation, using accessible language and tailored information for stakeholders with differing levels of HTA experience, and providing information in a range of formats such as examples and case studies.

Develop a user-friendly data-driven dashboard that makes it easier to find out about HTA processes, outcomes and performance.

Recommendation 24. Developing a stakeholder engagement framework

Co-design a stakeholder engagement framework to describe how and why engagement with stakeholders is used across all HTA processes, from horizon scanning to post-market reviews.

Recommendation 25. Improving the involvement of consumers in HTAs

Actively engage consumers across the HTA continuum, including by offering support and training.

Update the Pharmaceutical Benefits Advisory Committee Guidelines to request information from sponsors of HTAs about how they engaged with consumers during pre-HTA processes including clinical trial design.

Recommendation 26. Developing an explicit qualitative values framework

Support HTA committees to develop, in consultation with stakeholders, explicit guidelines and communications on the elements (beyond clinical effectiveness, cost-effectiveness and financial impact) they consider.

CHAPTER 7: ENHANCING REAL-WORLD DATA AND REAL-WORLD EVIDENCE FOR HTA

Recommendation 27. Governance and strategic oversight of real-world data to support HTAs

Develop an Australian framework to optimise timely access to relevant real-world data for HTA, covering enabling systems, pathways and evaluations, and research the collection and use of real-world data for HTA. This framework should:

- be co-designed by a multi-stakeholder advisory group that reports to the Australian Government, and oversees the implementation of the framework and related activities
- include a strategy to increase confidence, awareness and acceptance of cross-jurisdictional and cross-sectoral real-world data access and use in HTA.

Recommendation 28. Data infrastructure to support HTAs

Develop a dynamic, enduring whole-of-government data infrastructure that:

- evolves over time, based on needs
- is internationally harmonised, flexible, scalable and transparent

As an initial step, prioritise mapping Australian real-world data collections that could meet HTA needs, and facilitate access for relevant stakeholders.

Recommendation 29. Inter-governmental data collaboration in standardised collection and sharing of health technology–related data

Promote state and territory government collaboration and participation in cross-jurisdictional data sharing to support nationally cohesive HTA. This should be facilitated by centralised data-sharing infrastructure and harmonisation of access to existing government-held real-world data collections.

Recommendation 30. Real-world data and real-world evidence methods development

With oversight by the multi-stakeholder advisory group, establish a multi-stakeholder coordinated approach to developing transparent evidence for HTA using best-practice methods that span data standardisation, standardised analytics and reporting.

Recommendation 31. Collecting and using real-world data to resolve uncertainty

Ensure early identification and/or configuration of data collections that could help resolve uncertainties when it is expected that an application is likely to result in a managed entry agreement.

Begin early exploration and negotiation to determine feasibility and resourcing requirements that would meet the intended purpose. Resourcing should be jointly funded by relevant parties, with all details resolved before entering into a managed entry agreement. In the case of ultra-rare diseases and other small populations, international collaboration in the collection of patient-level data should be undertaken, where possible.

CHAPTER 8: METHODS FOR CONFIDENT DECISIONS

Recommendation 32. Creating a framework for Population, Intervention, Comparator, and Outcome – or PICO – scoping to support HTA submissions

Establish a framework to govern how Population, Intervention, Comparator, and Outcome (PICO) scoping and engagement occurs (the Australian Government should work with stakeholders to achieve this). The framework should establish circumstances where comprehensive PICO scoping would add value to HTA processes. The framework should ensure that criteria that are important to patients and clinicians are appropriately considered while also avoiding adding time or complexity to the HTA.

Recommendation 33. Methods for assessing consumer evidence

Support the development of updates to the Pharmaceutical Benefits Advisory Committee's and Medical Services Advisory Committee's guidelines, assessment methods, public summaries and other explanatory materials. Updates should be clear about how to integrate consumer evidence (research into patients' needs, preferences, experiences and perspectives) and consumer inputs arising from engagement processes (see Chapter 6) into HTA processes.

Recommendation 34. Overarching principles for adopting methods in Australian HTAs

Support the adoption of overarching principles for the methods used in Australian HTAs to ensure that decision-makers have the best possible evidence available and sponsors and evaluators understand preferred methods and approaches.

Recommendation 35. Methods for assessing non-randomised and observational evidence

Support the development of updates to methods for using non-randomised and observational evidence that are in line with the overarching principles for adopting methods in Australian HTAs.

Recommendation 36. Methods for assessing surrogate end points

Support the development of additional methods for using surrogate end points in HTAs that align with the overarching principles.

Recommendation 37. Methods preferred by decision-makers

Support the generation of a curated list of methodologies preferred by decision-makers.

Recommendation 38. Therapies that target biomarkers (e.g. tumour-agnostic cancer therapies and therapies that target cells with particular gene alterations)

Support the development of further guidance on methods for assessing tumour-agnostic therapies, genomic technologies and gene therapies.

Recommendation 39. Discount rate

Support reduction of the base case discount rate to no lower than 3.5% for health technologies that have upfront costs and benefits that are claimed to accrue over a long period (such as gene therapies and some vaccines).

Comment by Ms Elizabeth de Somer, Member Nominated by Medicines Australia: 'The industry recognises the movement in the discount rate in the recommendation and maintains that the base case discount rate should be reduced to 3.5% for all health technologies and 1.5% for those medicines where the benefits accrue over a longer time.'

Recommendation 40. Comparator selection

Support updates to the Pharmaceutical Benefits Advisory Committee Guidelines to clarify what alternative therapy should be selected as the main comparator in submissions for health technologies with multiple alternative therapies. The updates should make clear that health technologies claiming non-inferiority to the main comparator can be more expensive than lower-cost alternatives when those alternatives are inferior or for other clinical reasons no longer considered alternatives.

Comment by Ms Elizabeth de Somer, Member Nominated by Medicines Australia: 'The industry recognises the importance of the updated PBAC Guidelines that provide clarity to the PBAC and maintains this would be strengthened with an alternative recommendation: The National Health Act includes an additional clause to clarify that, in subsections 101(3A) and (3B), in having regard to the alternative therapy or therapies for the relevant patient population and any sub-populations, the Committee must consider the therapy or therapies most likely to be replaced in clinical practice.'

Recommendation 41. Cost-minimisation submissions

Investigate mechanisms to differentiate cost-minimisation submissions based on the proportionate benefit and relative cost.

Recommendation 42. Valuing and pricing

Conduct research to understand if and where it may be reasonable for HTA committees to accept higher prices for health technologies than are currently accepted.

Recommendation 43. Environmental impact reporting

Investigate options, in consultation with industry and stakeholders, for reporting environmental impacts in the assessment of health technologies.

CHAPTER 9: SUPPORTING ARCHITECTURE FOR HEALTH TECHNOLOGY ASSESSMENTS

Recommendation 44. Identifying therapeutic areas of high unmet clinical need

Develop a process and criteria to support ongoing identification of therapeutic areas of high unmet clinical need.

Recommendation 45. Identifying therapies to address therapeutic areas of high unmet clinical need

Develop a process for identifying high added therapeutic value health technologies that may address identified high unmet clinical need.

Recommendation 46. Proactive pre-HTA processes supporting the introduction of identified health technologies for high unmet clinical need

Establish arrangements that bring key stakeholders together to discuss how to bring forward timely development and lodgement of HTA submissions for identified health technologies that address identified high unmet clinical need.

Recommendation 47. Horizon scanning

Establish and resource an Australian horizon scanning function that improves stakeholder engagement in considering the implications of new and emerging health technologies and support healthcare forward planning and priority setting by healthcare payers.

Recommendation 48. Mechanisms for continuous review and improvement

Design and establish (in consultation with stakeholders) a program arrangement that supports the continuous review and updating of HTA policy and methods that support of the core pillars of the National Medicines Policy.

Recommendation 49. HTA evaluation workforce

Develop education programs and/or training activities to enhance HTA workforce competency and capability.

Progress reforms to support uptake of work-sharing arrangements.

Recommendation 50. Supporting architecture resourcing

Appropriately resource (in quantity and alignment) the Department of Health and Aged Care to implement agreed recommendations arising from this Review, including new activities and improvements to existing functions.

Chapter 1: Introduction

Policy context

Australia's health system provides universal access to high-quality, safe, effective and appropriate health care through a range of government funding programs, including the Pharmaceutical Benefits Scheme (PBS) and Medicare. Australian citizens have come to expect that this will include timely access to new health technologies, including innovative medicines and vaccines, that may further improve health care and health outcomes.

The National Medicines Policy (NMP)[12] is the overall framework for the use of medicines in Australia. It has four central pillars that seek to ensure:

- that individuals and the community have equitable, timely, safe and reliable access to medicines and medicines-related services at a cost they can afford
- medicines meet the required standards of quality, safety and efficacy
- quality use of medicines and medicines safety
- a collaborative, innovative and sustainable medicines industry and research sectors with the capability, capacity and expertise to respond to current and future health needs.

Health technology assessment (HTA) plays a central role in meeting the objectives of these pillars. The Review provided a significant and timely opportunity to examine Australia's approach to HTAs to determine what is working effectively, how to improve the current settings and how to futureproof the system to best meet the opportunities and challenges of the future.

How the Review was conducted

The Review was one of the key commitments in the *2022–2027 Strategic Agreement between the Commonwealth and Medicines Australia.*[13] The Minister for Health and Aged Care appointed the following Reference Committee to conduct the Review.

[12] DHAC (2022) *National Medicines Policy 2022*.

[13] DHAC (2022) *Strategic Agreement between the Commonwealth of Australia and Medicines Australia 2022-2027*.

Table 1: HTA Review Reference Committee membership

Member	Role on the Reference Committee
Adjunct Professor Debora Picone AO	Independent Chair
Dr Dawn Casey PSM	Patient representative
Ann Single	Patient representative
Professor Andrew Wilson AO	Chair of the Pharmaceutical Benefits Advisory Committee
Professor Andrew Roberts AM	Clinical/scientific representative
Elizabeth de Somer	Member nominated by Medicines Australia
Adjunct Professor Adriana Platona PSM	Member nominated by the Australian Government

**** Before Elizabeth de Somer's appointment to the Reference Committee, John Young was Medicines Australia's nominated member. He stepped down from 7 March 2023 due to taking up another position.***

The Reference Committee developed the Terms of Reference for the Review, met regularly and made an early decision to be open and inclusive with stakeholders throughout the Review.

Consultation

Extensive consultations were undertaken to support the Review and develop reform proposals. These included:

- **Two public consultations:** the first consultation closed in June 2023 and received 114 submissions, which included responses to an online survey, emailed submissions and online video forums with the Reference Committee. The second consultation sought feedback on options for reform and closed in February 2024. It received 139 written submissions and additional feedback through three online workshops and one in-person workshop. Reports on the consultations and submissions are published on the Department's website and Consultation Hub.
- **Deep dives with stakeholders:** these allowed the Reference Committee to gain an in-depth understanding of specific complex topics, issues, challenges and opportunities for HTA. A total of 26 deep dives were held, with 116 participants representing industry, consumers and patients, clinicians, First Nations people, and state and territory governments.

The quantity and quality of the responses to the Review's consultations are clear demonstrations of how important the HTA processes are to many stakeholders. These include individuals, families and carers; the Commonwealth; state and territory governments and regulatory agencies; non-government organisations; health practitioners; consumer organisations (including not-for-profits); the private and public health sectors; industry (including pharmaceutical, software and medical technology,

and service delivery industries); researchers and academics; health educators (including higher education and professional training bodies); health professional organisations and other health-related agencies; the media; and the general community.

The Review would like to thank all those who shared their aspirations, insights and knowledge – their input has been invaluable.

Expert analysis

The Review commissioned expert analysis so that the Reference Committee and stakeholders were informed by papers analysing contemporary research, relevant methodologies and purchasing practices used by comparable international jurisdictions, and their applicability to the Australian context. Drafts of these papers were published in advance of the second consultation. The expert papers and organisations that prepared them are as follows:

Adelaide Health Technology Assessment

- Paper 1. International Health Technology Market Approval, Funding and Assessment Pathways
- Paper 2. Horizon Scanning and Early Assessment
- Paper 3. HTA Methods: Determination of Population, Intervention, Comparator, and Outcome (PICO)
- Paper 4. Clinical Evaluation Methods in HTA

Centre for Health Economics Research and Evaluation

- Paper 5. HTA Methods: Economic evaluation
- Paper 6. Funding and purchasing decisions: Managing Uncertainty

Centre of Research Excellence in Medicines Intelligence

- Paper 7. Optimising the availability and use of real world data and real world evidence to support health technology assessment in Australia

The Department of Health and Aged Care

- Paper 8. Australian market authorisation, funding and assessment pathways and timelines
- Paper 9. Emerging Health Technologies

The structure of this report

After the preface, executive summary and overview of the Australian health system and HTAs, chapters 2 to 9 introduce the topic, give a contextual overview, consider the issues, and present the Review's findings and analysis, including recommendations.

- Chapter 2: Overview of Australia's health system and HTA
- Chapter 3: Providing more equitable access to under-represented patient groups
- Chapter 4: Streamlined pathways for more timely access
- Chapter 5: Policies, methods and processes supporting the translation of HTA recommendations into patient access
- Chapter 6: Transparency and stakeholder involvement
- Chapter 7: Enhancing real-world data and real-world evidence for HTA
- Chapter 8: Methods for confident decisions
- Chapter 9: Supporting architecture for health technology assessments

Chapter 2: Overview of Australia's health system and HTA

Australia's health system and access to health technologies

Australia's health system has helped to deliver better health outcomes on many measures for Australian citizens compared to other OECD countries.[14] Australia's mortality rates from preventable and treatable causes are among the lowest in the world, and Australians' life expectancy at birth (currently 83 years) is the fifth highest.[15]

The health system has contributed to these outcomes through funding schemes that provide universal access to a wide range of health services, public hospitals and technologies at low or no cost to citizens. These funding schemes include Medicare, the PBS, the National Immunisation Program (NIP) and the Life Saving Drugs Program (LSDP).

Committing taxpayer funds to these schemes is part of the Australian Government's broader goal of improving living standards for all citizens. Administration of Australia's healthcare funding schemes is guided by various policies.

The main policy framework that guides the provision of access to health technologies is Australia's NMP.[16] Its vision is 'to achieve the world's best health, social and economic results for all Australians and to do this through a supportive medicines policy'.[17]

To that end, the NMP has three key aims:

- to provide equitable, timely, safe and affordable access to a high-quality and reliable supply of medicines and medicines-related services for all Australians
- to ensure medicines are used safely, optimally and judiciously, with a focus on informed choice and well-coordinated person-centred care
- to support a positive and sustainable policy environment that drives world-class innovation and research, including translational research, and the successful development of medicines and medicines-related services in Australia.[18]

[14] See p68-69 in OECD (2023) *Health at a Glance*.

[15] WHO (2020) *Life expectancy at birth (years)*.

[16] Department of Health and Aged Care (DHAC) (2022) *National Medicines Policy 2022*.

[17] DHAC (2022) *National Medicines Policy 2022*.

[18] DHAC (2022) *National Medicines Policy 2022*.

For many years, Australia's funding schemes have supported these aims by providing Australian citizens with universal access to the most effective health technologies for the prevention, management and treatment of medical conditions.

Health expenditure and funding

Australia's health outcomes are achieved at a significant cost to the taxpayer. In 2022, Australia had the 11th highest health spending per capita among Organisation for Economic Co-operation and Development (OECD) member nations.[19] This has grown over time, driven by increased government investment and greater use of the health system by Australia's aging population.

Compared to international counterparts, Australia gets good value for its healthcare spend. Recent research by the Productivity Commission estimates that Australia has the third-highest healthcare sector productivity among 28 high-income countries.[20] Quality improvements have been one of the biggest contributors to productivity growth. Policies that maintain the sustainability of health funding programs and ensure rational decisions about which health technologies to fund have also had an important impact. These policies have successfully expanded access to new treatments, while managing growth in expenditure.

What is HTA and why is it important?

To achieve the best possible health outcomes, Australia's public health system must deliver the most effective treatments, when they are needed, and at a cost that individuals and the community can afford.

Decisions to make health technologies available under Australia's universal access schemes have an enormous impact on the lives of Australians. They affect the choices patients and their treating clinicians make about treatment. They determine whether patients receive the best available treatment for their circumstances, which can have life-changing consequences for them and their carers. They also have far-reaching impacts on the Australian community and represent a large investment of taxpayer funds.

To ensure decisions to fund health technologies through Australia's universal access schemes are in Australians' best interests, decision-makers need to be confident that they will work as well as, or better than, what Australians already have access to, and Australians will be better off, overall, if they are funded.

[19] See Figure 7.4 p157 in OECD (2023) *Health at a Glance*.

[20] See p3 in Australian Government Productivity Commission (2024) *Advances in measuring healthcare productivity*.

HTA is the process decision-makers follow to reach the above conclusions. HTA evaluates the best available evidence about the quality, safety, efficacy, cost-effectiveness and total costs of a health technology.

The main outputs of an HTA are conclusions about how well the health technology is likely to perform compared to the existing standard of care, and the cost to the taxpayer of claimed improvements to health outcomes. Importantly, the HTA processes also help decision-makers understand how confident they can be that a health technology will perform as claimed based on the available evidence.

Australia was one of the first countries in the world to use HTAs to inform decisions about what health technologies would be covered under its universal access schemes. The HTA system was first introduced for the PBS in 1993 and for Medicare in 2007. It is one of the main reasons the PBS has been able to continually increase access for patients while managing growing expenditure.

How does Australia do HTAs?

Decisions about which health technologies to include in Australia's universal access schemes are complex. This is because human health and the practice of medicine and health care are complicated, and increasingly innovative health technologies that address areas of unmet need are becoming available.

To make these decisions, the Government relies on submissions from industry and advice from independent expert committees comprising medical practitioners, health professionals, health economists, other experts and consumer representatives. The committee members are appointed to be the pre-eminent source of advice to the Government on decisions about which health technologies to subsidise, and for whom, at what cost and under what circumstances. They use HTAs to inform their advice.

HTA processes in Australia involve evaluating scientific evidence to determine the quality, safety, efficacy, cost-effectiveness and total costs of health-related goods and services. HTAs allow the estimation of the changes in health outcomes arising from the introduction of a new product into the Australian system relative to its costs to the health system if it is funded.

Why is Australia's approach to HTAs being reviewed?

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 been clear and, for the most part, has greatly exceeded the impost on public funds – they provide a positive return on investment.

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 may treat lethal and severely disabling conditions where previously no effective treatment was available. Some can do this with a single treatment, providing flow-on benefits to the health system, carers, families, productivity and society.

For Australian citizens to benefit from the latest health technologies, decision-makers need to be able to conclude, in a timely way, that funding them is in the best interests of Australian citizens.

However, making these decisions is becoming increasingly challenging. The health technologies emerging today, and that are likely to emerge in the near future, are increasingly diverse and complex.

Patients, clinicians, industry and the Government have expressed concern that funding new health technologies in Australia takes too long and that HTA processes need to be futureproofed for the latest emerging technologies.

Many emerging technologies do not fit neatly into the treatment categories for which Australia's funding and assessment processes were designed.[21] Some are being developed to treat rarer conditions and potentially deliver longer-term benefits. For many emerging therapies, the evidence needed to conclude how well they are likely to perform does not allow decision-makers to be as confident as they were about medicines that emerged in the 1990s and 2000s. Several emerging therapies also have high risks (including severe adverse effects), requiring more resource-intensive implementation than traditional therapies.

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 more than $57,000. By 2020, the most expensive was $110,000. Today, it is a little over $2.5 million, meaning the most expensive medicine on the PBS today is 100 times more expensive than in 2010.

These issues have been raised in other inquiries and reform processes, including the *2022–2027 Strategic Agreement between the Commonwealth and Medicines Australia*,[22] the NMP Review[23] and the House of Representatives Standing Committee for Health,

[21] See p11–12 in DHAC (2023) *Emerging health technologies*, Health Technology Assessment Policy and Methods Review.

[22] DHAC (2022) *Strategic Agreement between the Commonwealth of Australia and Medicines Australia 2022-2027*.

[23] DHAC (2022) *National Medicines Policy - consultation on the revised NMP*, DHAC Consultation Hub DHAC website.

Aged Care and Sport's 'Inquiry into approval processes for new drugs and novel medical technologies in Australia' (The New Frontier inquiry).[24]

The Review was undertaken to further the work of those processes and deliver a set of recommendations for reform to the Australian Government.

How long does Australia take to provide access?

The Review examined multiple studies on how long it takes health technologies to gain public or universal funding in Australia and overseas. It found that Australia's processes rank between the top and middle of comparable OECD countries, depending on the timeliness measure.[25,26,27]

Table 2: Average time for HTA decision-making and reimbursement of pharmaceuticals in Australia, ranked against comparable OECD countries

Author, study	Measure	Result	Rank
Centre for Innovation in Regulatory Science, *Review of HTA outcomes and timelines in Australia, Canada and Europe 2018-2022*	Time taken from regulatory approval to HTA recommendation (2022)	3 months	1st out of Australia, Canada, England, France, Germany, Netherlands, Poland, Scotland, Sweden
Medicines Australia, *Medicines Matter 2022* report	Average time from registration to reimbursement (2016–2021)	15 months (OECD average 13 months)	11th out of 20 OECD countries
Pharmaceutical Research and Manufacturers of America, *Global Access to New Medicines Report*	Time from global first launch to public reimbursement	47 months (G20 average 46 months)	10th in G20

For medicines that were added to the PBS in 2021 and 2022, the shortest time between Therapeutic Goods Administration (TGA) registration and PBS subsidy was 2 months. However, the majority of new medicines took significantly longer than this – the

[24] Australian Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

[25] Sola B, Wang T and McAuslane N (2023) *R&D Briefing 89: Review of HTA outcomes and timelines in Australia, Canada and Europe and the UK 2018-2022*, Centre for Innovation and Regulatory Science.

[26] Medicines Australia (2022) *Medicines Matter 2022: Australia's Access to Medicines 2016-2021*.

[27] Pharmaceutical Research and Manufacturers of America *(2023) Global Access to New Medicines Report*.

median time to achieve PBS listing after TGA registration was 21 months. For new medicines that improved health outcomes, the median time was 22 months.[28]

Deeper examination of the data revealed that one of the main reasons most medicines did not achieve PBS listing in the shortest possible time frame was that multiple submissions to the PBAC were required to achieve a positive recommendation. Another key reason was underuse of processes that allow concurrent processing (parallel processing) of TGA and PBAC applications. As such, the Review focused on improving the first-time success rate of health technology funding applications and incentivising use of parallel processing.

For highly specialised therapies (HSTs) funded through public hospitals, health technologies funded through the LSDP, and vaccines funded through NIP, additional steps in the process mean they take significantly longer to be funded than PBS medicines. The Review, therefore, also focused on streamlining the pathways to funding through these schemes.

[28] See p36 in DHAC (2023) *Australian market authorisation, funding and assessment pathways and timelines*, Health Technology Assessment Policy and Methods Review.

Chapter 3: Providing more equitable access to under-represented patient groups

Introduction

Some population groups, such as First Nations people and children, are under-represented in the delivery and access to vital medicines. Providing equitable access to these groups is complex and requires holistic solutions integrated across the HTA continuum.

Chapter 3 provides the Review's consideration and recommendations for:

- improving engagement and consultation with First Nations people in HTA processes
- improving access to medicines for paediatric patients.

Chapter 3.1: Creating an equitable system for First Nations people

Introduction and context

While there are mechanisms to promote equitable access to medicines for First Nations people, this population group lacks formal and routine involvement in HTAs. The lack of embedded First Nations health representatives and consideration of First Nations perspectives and health outcomes is contributing to health inequity. Stakeholders noted that several medicines integral to the health and wellbeing of First Nations people are not listed on the PBS. Data revealed disparities in access to medicines for First Nations people, with significantly lower expenditure on medicines for this population group.

Chapter 3.1 explains the Review's consideration of these issues and its recommendations for more formal arrangements to ensure impacts on First Nations people are fully considered at all stages of HTAs.

Under current arrangements, processes for involving First Nations people in HTA processes are the same as those for all Australians. Stakeholders from all impacted groups can make submissions for the funding of new health technologies or to provide commentary on medicines being considered by the PBAC.

When making a submission, sponsors have the option to include details regarding the impact of the medicine on health equity, including the impact on First Nations people. However, the sponsor has discretion about whether to include this information, so it is often left out. Therefore, the PBAC does not consistently consider the impact on health equity and outcomes for First Nations people it in its decision-making.

The Commonwealth has committed to delivering objectives in the National Agreement on Closing the Gap (the National Agreement).[29] The National Agreement was developed in partnership between all Australian governments and the Coalition of Peaks, a representative body of Aboriginal and Torres Strait Islander organisations. As part of the National Agreement, governments committed to improving engagement with First Nations people, agreeing to:

> Ensure when governments are undertaking significant changes to policy and programs that primarily impact on Aboriginal and Torres Strait Islander people, they engage fully and transparently. Engagements should be done in a way where Aboriginal and Torres Strait Islander people: have a leadership role in the design and conduct of engagements; know the purpose and fully understand what is being proposed; know what feedback is provided and how that is being taken account of by governments in making decisions; and are able to assess whether the engagements have been fair, transparent and open.[30]

In line with the priority reforms under the National Agreement, it is important that the way government engages with First Nations people across the HTA continuum is given close consideration.

To minimise barriers to First Nations people accessing PBS pharmaceuticals, a range of strategies are used, including cost-recovery fee waivers for submissions to the PBAC for medicines for First Nations people.

In recognition of their specific health needs, a range of over-the-counter medicines (and some low-cost prescription-only items) are subsidised through the PBS specifically for prescribing for First Nations people. Special supply arrangements under section 100 of the *National Health Act 1953* (NHA) have been implemented to allow PBS medicines to be provided to primary healthcare services, such as Aboriginal Community Controlled Health Services (ACCHSs), that treat First Nations people in remote locations. These medicines are provided at no cost, by suitably qualified and approved health service professionals, without the need for a PBS prescription.

Despite the measures aimed at minimising barriers to access for First Nations people, the per-person pharmaceutical expenditure and PBS-subsidised pharmaceutical expenditure still show disparities. The per-person spend on pharmaceuticals for First

[29] Australian Government (2020) *National Agreement on Closing the Gap*.

[30] See 59f, Priority Reform Three – Transforming Government Organisations in Australian Government (2020) *National Agreement on Closing the Gap*.

Nations people by the Australian Government in 2015–16 was $537, 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 people and 48% for non-Indigenous people. This represents an average expenditure by the Government of $167 per person for First Nations people, compared to $427 per person for non-Indigenous Australians (2015–16 data).[31]

What we heard:

> 'Funding and purchasing are possibly the most acute issues for NACCHO [the National Aboriginal Community Controlled Health Organisation] within HTA currently. The medicines that our sector would like listed are commonly low-cost items for a relatively small population. Even with medicines and technologies with a high degree of uncertainty, the low cost and small population creates a very low financial risk for government. We therefore propose price policy should be much more explicitly addressed in relation to listing for Aboriginal and Torres Strait Islander health technologies on the MBS and PBS. Specifically, the payer should have structured means to pay a higher price for an item that will disproportionately benefit Aboriginal and Torres Strait Islander people. Such an approach is a practical way of meeting equity needs expressed in the National Medicines Policy.'
>
> **Consultation 1 submission: National Aboriginal Community Controlled Health Organisation (NACCHO)**

As part of the consultation process, stakeholders raised concerns about ad hoc engagement of First Nations people and ACCHSs. Stakeholders noted the lack of a dedicated body that fosters relationships or systematically seeks advice from First Nations people. Stakeholders noted the absence of a proactive approach to identifying unmet clinical needs and appropriate treatment options was disproportionately affecting the proper care of First Nations people.

Stakeholders said many medicines that are integral to the health and wellbeing of First Nations people are not listed on the PBS. Stakeholders noted that this was due in part to the small patient population of First Nations people, compared to the wider Australian population. This has resulted in less commercial incentive for sponsors to make submissions. Stakeholders also felt there was inadequate support for groups to

[31] Australian Institute of Health and Welfare (2020) *National Indigenous Australians Agency, Aboriginal and Torres Strait Islander Health Performance Framework: 3.15 Access to prescription medicines*.

bring forward applications for funding for these medicines. This is impacting the availability of subsidised access to prescription and over-the-counter medications for First Nations people.

Stakeholders also raised that sponsors are not required to identify the needs of particular population groups, such as First Nations people, when submitting medicines for consideration by the PBAC. This is preventing the PBAC from considering consistent advice and evidence from sponsors about potential impacts on health equity and outcomes for First Nations people.

Following the initial consultation, the Review sought additional feedback options to:

- establish decision-making partnerships with First Nations people
- provide a dedicated resource for HTA submissions and education to support First Nations people and organisations.

What we heard:

The involvement of First Nations people and consideration in HTA processes received strong support across Consultation 2. The proposed options for reform mostly addressed the issues raised, according to:
- 64% of patient and consumer groups
- 86% of pharmaceutical or medical technology companies
- 67% of industry or peak body representatives
- 50% of clinicians.

85% of stakeholders supported having First Nations representative/s on the PBAC to speak specifically to First Nations people's health.

82% of stakeholder groups thought having a dedicated resource to assist organisations representing First Nations people would have a positive impact on themselves or their organisation.

Stakeholders also supported using a proactive approach in HTA processes to identify unmet needs and potential therapies to meet these needs, based on governments' commitments in the National Agreement.

Representative responses to Consultation 2

In its response to Consultation 2, NACCHO noted that an HTA should consider the societal and equity principles, and distributional impacts, of treatments. This is particularly relevant to First Nations people who have distinct health paradigms and value perspectives. NACCHO reiterated that subjectivity and perspectives need to be considered in patient safety, effectiveness and cost of medicines. A specialist's

judgement of effectiveness may be different to the view of a First Nations person. For example, limited formal data may be available about the safety of a traditional First Nations medicine, despite its clear utility for a community. Therefore, the distinct perspectives of First Nations people bring to the concept of effectiveness is balanced with safety and cost.[32]

Findings

There is a lack of formal and routine involvement of a First Nations health representative in decision-making, particularly in the PBAC and the Medical Services Advisory Committee (MSAC). Decision-making processes and recommendations do not take into account the full consideration of First Nations people's health outcomes. This is contributing to health inequity.

Conclusion

The Review has recommended that HTA advisory committees would benefit from having more formal First Nations engagement arrangements, such as including First Nations representatives, or a professional with distinct and recent expertise and experience in First Nations people's health issues, on the PBAC. This would assist in ensuring that potential health and social impacts on First Nations people were considered when making decisions about listing or delisting medicines on the PBS.

The Review also recognised that First Nations people must be involved across the HTA system to proactively identify the self-determined unmet needs of, and new and emerging therapies for, First Nations people. This would align with priorities outlined in the Government's commitments in the National Agreement.[33]

Objectives of recommendations

The Review's recommendations aim to reduce health inequity for First Nations people by enhancing access to appropriate health technologies to meet their specific needs. This will be achieved by ensuring adequate and appropriate consideration of their health, wellbeing and access needs through partnerships and shared decision-making.

Recommendations

Recommendation 1. Creating a more equitable system for First Nations people

The Review recommends that the Australian Government:

a. establish a First Nations Advisory Committee, reporting to the Pharmaceutical Benefits Advisory Committee (PBAC) and the Medical Services Advisory

[32] NACCHO's submission to Consultation 2.

[33] Australian Government (2020) *National Agreement on Closing the Gap*.

Committee (MSAC), to contribute to decision-making across the healthcare continuum, including:

i. in line with the priority reforms under the National Closing the Gap Agreement 2020, developing a priority list of indications, in partnership with Aboriginal Community Controlled Health Services (ACCHSs), for First Nations people with high unmet clinical need (HUCN)
ii. developing an active horizon scanning process to identify therapies with promising high added therapeutic value for indications on the priority list. This could include new therapies or new indications for 'repurposing' existing therapies
iii. advising on proactive submission requests for therapies on the priority list, prioritising those that address areas of unmet clinical need and gaps in access.

b. include representation on the PBAC for First Nations people, including individuals and/or professionals with current expertise and experience in health issues relating to First Nations people, and who are actively engaged in this area. The role should speak to the specific benefits to First Nations people and be accountable for decision-making on behalf of First Nations people
c. require sponsors' submissions to include consideration/assessment of the impact on health outcomes for First Nations people. This will enable more meaningful and informed decision-making by the PBAC and the MSAC
d. develop, in collaboration with ACCHSs, a mechanism to recognise the additional benefit of therapies to First Nations people for the priority health areas listed under the *National Agreement on Closing the Gap (2020)*
e. develop First Nations HTA criteria. This would support timely listing of medicines with specific health benefits for First Nations people. This could include fit-for-purpose criteria to allow decisions to be made on a range of population health outcomes, not just one clinical end point
f. resource the Department of Health and Aged Care to assist organisations representing First Nations people to submit and engage in HTA submissions. This would include providing education, support and funding to organisations to develop submissions and be set out in the *Stakeholder Engagement Framework* (See Chapter 6.2).

Chapter 3.2: Improving access to medicines for paediatric patients

Introduction and context

There is a lack of therapies for paediatric patients. This is mainly attributed to challenges generating clinical data on paediatric patients for regulatory approval and consequently reimbursement. The return on investment for paediatric indications often

does not offset the costs associated with the complexities relating clinical trials for paediatric patients. This is a barrier to the development and listing of specific medicines for paediatrics patients.

Chapter 3.2 explains the Review's consideration of these issues and recommendations to create more equitable access for paediatric patients.

As most submissions to the PBAC are for medicines to treat adult patients, listings specific to paediatric patients comprise a small proportion of the PBS. Clinical trials are often complex and expensive. Trials in paediatric patients are associated with additional challenges including ethical considerations, a smaller pool of eligible participants, required adaptations to protocols, and additional research infrastructure to accommodate children's development. Consequently, the return on investment for paediatric therapies and paediatric indications is often inadequate to offset the costs associated with their development and approval, and they represent a much smaller market for pharmaceutical sponsors.

During The New Frontier inquiry, stakeholders expressed that more incentives were required to increase the number of therapies to treat diseases in smaller populations, such as paediatric patients, and noted that challenges with conducting clinical trials was a major barrier in the approval of new drugs for these populations.[34] The inquiry recommended that the Review reassess relevant aspects of HTA processes to ensure there are future pathways for treatments and therapies that do not fit neatly into the current system. It also said, 'It is imperative that appropriate clear pathways are considered for inclusion for paediatric medicines and technologies'.[35]

When recommending medicines for listing, the PBAC accepts that products included in the Australian Register of Therapeutic Goods (ARTG) have adequate safety and efficacy to allow marketing in Australia for the specific therapeutic indications for which they are TGA-registered. Generally, the PBAC will not recommend listing a product on the PBS for indications beyond those approved by the TGA. While the PBAC will not directly contradict TGA decisions, it has in instances omitted age from PBS restrictions, allowing broader access for children than that approved by the TGA.

Findings

Stakeholders noted that the proposed options for reform contained in Consultation 2 lacked pathways and approaches to specifically improve access to, and availability of, paediatric medicines.[36] However, stakeholders considered that certain proposed

[34] See comment from the Association of Australian Medical Research Institutes on p227 as an example, Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier* inquiry.

[35] Recommendation 29, Australian Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

[36] See Asthma Australia's submission to Consultation 2 for a representative view on this observation.

options – such as developing a priority list of areas of high unmet clinical need (HUCN), proactively inviting and incentivising sponsor submission, and arranging bridging funding for earlier access to therapies addressing HUCN – may help to improve access to medicines for paediatric patients.[37]

Age-agnostic PBS listings have not been applied in a systematic manner, which is likely to have contributed to unequal access for paediatric patients across the PBS.

It was also noted that a lack of clinical trials in paediatric populations is a fundamental barrier to improving access for paediatric patients, though the policies and regulations that guide clinical trial development are beyond the scope of the Review.

Given the challenges in generating trial data in paediatric populations, obtaining regulatory approval may be difficult for pharmaceutical sponsors in many instances. And while the TGA's processes are beyond the Review's scope, increased collaboration between regulatory and HTA bodies to address some of the challenges with paediatric trial data could facilitate timely regulatory approval through to HTA.

Conclusion

A systematic approach to implementing new PBS listings without age restrictions would create more equitable access for paediatric patients.

The Review's recommendations that set out arrangements for identifying, screening and inviting health technology submissions for public subsidy that address areas of HUCN will also support the introduction of health technologies in Australia that address the treatment needs of paediatric patients.

Objectives of recommendations

The Review's recommendations are intended to improve access to PBS-listed medicines for paediatric patients and facilitate regulatory approval and reimbursement of medicines for paediatric indications.

Recommendations

Recommendation 2. Providing equitable access to medicines for children and young people

The Review recommends that the Australian Government:

a. formalise a systematic approach for new listings on the Pharmaceutical Benefits Scheme (PBS) to be agnostic of age. This would exclude listings where the Pharmaceutical Benefits Advisory Committee's (PBAC's) reasons for recommending restricted access are based on important safety, effectiveness, cost-effectiveness or quality use of medicines considerations

[37] See Asthma Australia's submission to Consultation 2 for a representative view on this matter.

b. identify, through consultation with stakeholders, existing PBS listings that could be amended to be agnostic of age
c. establish a working party to develop guidance on evidentiary requirements for extending the use of therapies registered with the Therapeutic Goods Administration (TGA) to paediatric populations. The working party should be jointly led by representatives of the TGA and the PBAC and include representation from industry and patient sectors.

Chapter 4: Streamlined pathways for more timely access

Introduction

Australia has several pathways for assessing health technologies for Australian Government funding. Each distinct funding and assessment pathway incorporates a range of processes and decision points, and involves several decision-makers and different stakeholders. These pathways and their component processes form a complex ecosystem, with numerous interdependencies and interactions between the different pathways.

The findings from the Review highlight that Australia's HTA and medicines funding systems are world class. The Review sought to identify areas to improve and optimise the systems and processes to enable them to keep providing Australians with world-class access to medicines while meeting the needs of Australians into the future.

This chapter covers the Review's analysis, findings and recommendations in relation to the issues and opportunities for reform of the ecosystem of funding and assessment pathways for health technologies seeking government funding. It includes analysis of the issues and opportunities for improvement in the current pathways and their component processes, as well as an overarching systemic view. The topics covered include:

- system level view of Australia's health technology funding and assessment pathways, including:
  - overarching systemic recommendations for health technology funding and assessment pathways and processes
- medicines applying for listing on the PBS, including:
  - a streamlined cost-minimisation analysis pathway for therapies applying for PBS listing
  - therapies with high added therapeutic value in areas of unmet clinical need applying for PBS listing, including processes for:
    - enhanced early resolution
    - case management of submissions
    - decoupling the TGA delegate's overview from the PBAC advice
  - alternative modelling and analysis types for disease areas
- vaccines applying for listing on the NIP
- highly specialised therapies (HSTs) and other therapies co-funded between the Australian and state and territory governments

- life-saving drugs for patients with ultra-rare diseases (Life Saving Drugs Program)
- the impact of reforms on timeliness: Joint performance targets.

As a framework for understanding the issues and opportunities for improvement, the Review's objectives included identifying features of HTA policy and methods that are working well, as well as those that:

- impact equitable access to health technologies
- impact timely access to health technologies
- detract from a person-centred health system
- create perverse incentives.

The Review aimed to leverage this understanding to create an implementable, sustainable set of recommendations for the Government that would tackle identified challenges and provide Australians with equitable, timely and safe access to affordable medicines. Additionally, the Review aimed to ensure that HTA policy and methods were equipped to evaluate emerging technologies into the future.

To achieve these goals, the Review developed overarching principles for reforms recommended for all HTA pathways and processes.

Overarching principles for reforms recommended for all HTA pathways and processes

1. Streamlined and simple

Streamline and simplify health technology funding and assessment processes by removing unnecessary complexity and redundancy.

2. Proportionate and fit-for-purpose

Restructure and develop health technology funding and assessment processes, including the level of evaluation, to be fit-for-purpose and proportionate to the level of risk, complexity and potential benefit of a therapy.

3. Unified and consistent

Align the health technology funding and assessment pathways, processes and committees, and remove duplication between them, in stages toward a single unified HTA committee and pathway approach. This approach should be designed to ensure that the health technology funding and assessment processes, pathways and committees support optimal patient care and do not fragment care pathways.

Chapter 4.1: Overview of Australia's health technology funding and assessment pathways

Introduction and context

Australia has a multi-payer health system that incorporates public and private health expenditure. Private funding of health care includes payments from individuals, as well as insurance companies. The responsibility for funding the public health system is shared between the Australian and state and territory governments. The Australian Government is directly responsible for funding medical services that are listed on the Medicare Benefits Schedule (MBS), and health technologies that are subsidised and funded through the PBS, LSDP, NIP and others. The state and territory governments are responsible for operating and administering public hospitals; however, funding responsibility is shared with the Australian Government. The funding arrangements between the Australian and state and territory governments are managed through the 2020–25 Addendum to the National Health Reform Agreement (NHRA).[38]

Before health technologies can be made broadly accessible to Australians, companies are usually required to seek TGA authorisation to supply them in Australia. The TGA assesses the health technology for safety and efficacy, in simple terms answering the questions 'is it safe?' and 'does it work/does it do what it claims to?'. This is called 'regulatory assessment' or 'health technology assessment for market authorisation'. After the TGA approves a medicine for the ARTG, doctors can write scripts for it and people are generally able to purchase it; however, this will be either at their own expense or covered by private insurance.

In Australia, many health technologies are subsidised or funded by the Government, meaning that Australians either do not have to pay or only pay a portion of the cost of medicines and other health technologies and services. The Government has a responsibility to the Australian public to ensure it is using public funds in an efficient and equitable way. So, before a health technology company can receive public funding for its health technology, it is assessed by expert advisory bodies such as the PBAC and the MSAC, or state and individual hospital's HTA bodies. These HTA advisory committees assess the clinical effectiveness and cost-effectiveness of a health technology compared to available alternatives. Put simply, they advise on the question, 'does it improve patient outcomes, safety or satisfaction at a reasonable cost that individuals and the community can afford?' This is referred to as 'HTA for reimbursement / government subsidy'.

[38] DHAC (2020) *2020–25 Addendum to the National Health Reform Agreement*.

Several different funding and subsidy programs underpin the public health system in Australia including:

- Pharmaceutical Benefits Scheme
- National Immunisation Program
- Life Saving Drugs Program
- Medicare Benefits Schedule
- PBS–MBS codependent technologies
- National Health Reform Agreement
- National Blood Arrangements.

Findings

The public funding program for a new health technology is determined by both the type of health technology and the patient care setting where the health technology will be delivered. In turn, the HTA pathway and advisory committee are determined by the funding program. As a result, therapies can go down different pathways and be evaluated by different HTA advisory committees, depending on where they will be provided to patients; for example, in a public hospital or a community pharmacy. This results in inconsistencies between the funding and assessment of health technologies that are delivered in different care settings.

Conversely, within the HTA and funding pathways, there is little difference between low-risk, simple submissions and high-risk complex submissions with respect to the amount of time and effort required for developing and evaluating the submission. This results in a lack of prioritisation of time and effort in the HTA system to achieve the most needed and useful clinical outcomes.

Conclusion

The funding and assessment pathways for health technologies in Australia detailed through chapters 4.2 to 4.6 are complex with many interdependencies and in some cases, duplication. Aligning the different pathways and processes as well as simplifying and streamlining the individual pathways can improve time to access and reduce burden on the system. Additionally, aligning the health technology funding and assessment to be appropriate and fit for purpose for the clinical need and clinical benefit of the therapy and complexity of the submission, will improve the sustainability and outcomes of the HTA system.

Objectives of recommendations

The recommendations in this chapter are designed as guiding principles for the reform and the development of all health technology funding and assessment pathways and processes, with the objectives of:

- improving time to subsidised access of health technologies by removing duplication and unnecessary steps
- improving time to subsidised access of health technologies by ensuring the effort of the health system is directed to where it is most needed
- improving consistency and certainty in the HTA system and processes, and making them easier for stakeholders to navigate
- improving equitable access, and ensuring that HTA and funding processes and pathways support optimal, continuous patient care.

Recommendations

Recommendation 3. Overarching recommendations for all HTA funding and assessment pathways and processes

The Review recommends that the Australian Government apply the following overarching principles to the restructuring and development of health technology funding and assessment processes and pathways:

a. Health technology funding and assessment processes, including the level of evaluation, should be fit-for-purpose and proportionate to the level of clinical benefit, clinical need, complexity and financial risk relating to the health technology submission
b. Health technology funding and assessment processes should be streamlined and simplified, with unnecessary complexity removed
c. Consistency and clarity should be created for all health technology submissions for Australian Government funding by developing a unified HTA pathway and committee approach, progressed in stages. This should start with better aligning the HTA pathways, and removing duplication between them. It should be designed to ensure that the health technology funding and assessment processes, pathways and committees support optimal patient care and reduce any fragmentation in care pathways (see 'Recommendation 4. Unified HTA pathway and committee approach for all applications for Australian Government funding for health technologies'). This would be supported by:
    i. the HTA pathway and committee being determined by the assessment requirement for the health technology
    ii. the assessment committee being able to recommend the most appropriate funding mechanism for optimal patient care.

Recommendation 4. Unified HTA pathway and committee approach for all Australian Government funding of health technologies

The Review recommends that the Australian Government develop, in consultation with stakeholders, a unified, national HTA pathway and committee approach for all health technology evaluation. The unified approach should include:

a. developing a unified HTA advisory committee approach, including changes to the committee composition and scope to ensure it is resourced to consider the breadth of different health technologies. Consideration should be given to the following options for achieving this:
    i. the feasibility of having a smaller core committee membership (for example, 6–10 core members), supplemented by other members drawn from a larger membership pool of different experts, as needed
    ii. the feasibility of having two committees, with the same approach, that have alternating meeting cycles
    iii. the most appropriate submission cycle duration and whether more flexibility or time is desirable and feasible.
b. a staged approach to implementation, with set review points along the stages to ensure changes are meeting objectives. Stages in this process should include the entire or components of:
    i. Recommendation 5. Triaging submissions
    ii. Recommendation 6. Expanding the advisory role of the Pharmaceutical Benefits Advisory Committee beyond the Pharmaceutical Benefits Scheme
    iii. Recommendation 11. Proportionate appraisal pathways to align the Australian Technical Advisory Group on Immunisation assessments with the level of risk and complexity of the product
    iv. Recommendation 14. Improving time to access life-saving drugs for patients with ultra-rare diseases (Life Saving Drugs Program).

Recommendation 5. Triaging submissions

The Review recommends that the Australian Government develop processes, in consultation with stakeholders, to enable triaging of submissions to determine the appropriate evaluation and appraisal mechanisms. The process for triaging should include:

a. submitting all HTA applications for Australian Government reimbursement using a 'single front door' approach.
b. developing a clear and transparent decision tool, such as a decision tree, to guide sponsors' nomination, and to triage selection of the most appropriate HTA pathway
c. establishing a triaging committee for example comprising of the chairs and deputy chairs of the Pharmaceutical Benefits Advisory Committee (PBAC), Medical Services Advisory Committee (MSAC), Australian Technical Advisory Group on Immunisation (ATAGI) and technical sub-committees
d. allowing sponsors to nominate their preferred or appropriate pathway based on their intended submission

e. giving the triaging committee responsibility for considering the submission's product type, risk, complexity, potential benefit and the sponsors nominated pathway to confirm an appropriate and proportionate:
    i. health technology funding and assessment pathway
    ii. HTA advisory committee and technical sub-committees
    iii. Population, Intervention, Comparator, and Outcome (PICO) scoping and engagement approach
    iv. HTA meeting date.

The triaging processes should facilitate 'Recommendation 3. Overarching recommendations for all health technology finding and assessment processes and pathways' and 'Recommendation 4. Unified HTA pathway and committee approach for all Australian Government funding of health technologies'.

Recommendation 6. Expanding the advisory role of the Pharmaceutical Benefits Advisory Committee beyond the Pharmaceutical Benefits Scheme

The Review recommends that the Australian Government, as a stage in the development of a unified HTA pathway (see 'Recommendation 4. Unified HTA pathway and committee approach for all Australian Government funding of health technologies'), expand the advisory role of the Pharmaceutical Benefits Advisory Committee (PBAC) to enable it to make HTA recommendations to the Minister for Health and Aged Care for a broader range of health technologies across different funding and subsidy programs. This could include:

a. medicines for inclusion on the Life Saving Drugs Program (see 'Recommendation 14. Improving time to access life-saving drugs for patients with ultra-rare diseases (Life Saving Drugs Program)')
b. co-dependent health technologies funded through the Medicare Benefits Schedule (MBS) and Pharmaceutical Benefits Scheme (PBS)
c. Highly specialised therapies funded through the 2020–25 Addendum to the National Health Reform Agreement (NHRA).

Chapter 4.2: Improved pathways for listing medicines on the PBS

Introduction and context

The PBS is the main subsidy program for medicines in Australia. It was established under the *National Health Act 1953* (NHA) and is funded by the Australian Government. Medicines that are subsidised through the PBS are listed on the Schedule of Pharmaceutical Benefits.

Generally, medicines listed on the PBS are dispensed by community pharmacies and used by patients at home. These are known as 'General Schedule' or 'section 85' medicines because they are dispensed under section 85 of the NHA. In some cases, where normal supply arrangements are not suitable, PBS medicines are supplied through special arrangements under section 100 of the NHA. For example, some medicines may require special storage or dispensing, specialist monitoring during treatment, or administration in a hospital outpatient setting.[39]

The NHA established the requirements for listing a medicine on the PBS, including that the Minister for Health and Aged Care cannot list any medicine on the PBS without a positive PBAC recommendation. The PBAC is an independent expert HTA advisory committee.

An HTA for medicines being considered for listing on the PBS involves several steps. Generally, it follows this process:

1. The sponsor (usually a pharmaceutical company) responsible for a medicine develops a submission in line with the PBAC Guidelines.[40]
2. The submission goes to the PBAC Secretariat at the Department.
3. The submission is evaluated, and an evaluation document (a commentary) is produced. For submission categories 1 and 2, and standard re-entry and facilitated resolution resubmissions, the PBAC evaluation groupsconduct the evaluation. For category 3 and 4 submissions, the Department does the evaluation.[41]
4. The sponsor is given the commentary for response or comment.
5. The PBAC Economic Subcommittee (ESC) considers the submission and sponsor's response, and prepares advice to the PBAC. The PBAC Drug Utilisation

[39] See p.24, Pharmaceutical Benefits Scheme in DHAC (2023) *Australian market authorisation, funding and assessment pathways and timelines*, Health Technology Assessment Policy and Methods Review.

[40] DHAC (2016) *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*. Pharmaceutical Benefits Scheme.

[41] See Section 4 Pharmaceutical Benefits Scheme in DHAC (2023) *Australian market authorisation, funding and assessment pathways and timelines*, Health Technology Assessment Policy and Methods Review.

Subcommittee (DUSC) may also consider and provide advice to the PBAC, where appropriate, for the HTA.

6. The sponsor is given a summary of the ESC (and DUSC) advice and prepares a response.
7. The PBAC appraises the submission, including the commentary, ESC advice and sponsor response, and makes a recommendation.
8. If the PBAC recommends that the medicine be listed on the PBS, the Minister for Health and Aged Care, or a delegate, can approve a PBS listing for a medicine with a financial impact of less than $20 million per annum.
9. Where the financial impact is greater than $20 million per annum, the Minister requires a Cabinet decision.

The PBAC has three main meetings, plus three intracycle meetings each year. The PBAC main meetings run on a 17-week cycle, with the submission due at week 0 and the PBAC meeting at week 17. Some steps occur outside the 17-week cycle. These include the issuing of notices of intent to lodge a submission, which are generally due four weeks before the submission is due, and receipt of minutes by applicants, which occurs 3–5 weeks after the meeting. These steps are followed by publication of public summary documents (PSDs) up to 16–18 weeks after the meeting.

There are nine different PBAC submission types, including four initial submission types, secretariat listings and four different resubmission pathways.[42]

Findings

Identifying features of HTA policy and methods that affect the time it takes for Australians to gain subsidised access to new medicines was a key objective of the Review. Research from the Centre for Innovation and Regulatory Science – HTA dock[43] shows that for comparable countries, Australia's time from regulatory approval (ARTG registration) to an HTA decision is the shortest of all countries in the study group. However, looking at the time it takes for Australians to access subsidised medicines relative to the comparable countries, as shown in *Pharmaceutical Research and Manufacturers of America (PhRMA) - Global Access to New Medicines Report*,[44] Australia sits in the median position, with several countries providing subsidised access sooner. So while the 17-week PBAC cycle is exceptionally short, there is a disparity between what the HTA system can technically achieve and what occurs in reality.

[42] See Section 4 Pharmaceutical Benefits Scheme in DHAC (2023) *Australian market authorisation, funding and assessment pathways and timelines*, Health Technology Assessment Policy and Methods Review.

[43] Sola B, Wang T and McAuslane N (2023) *R&D Briefing 89: Review of HTA outcomes and timelines in Australia, Canada and Europe and the UK 2018-2022*, Centre for Innovation and Regulatory Science.

[44] Pharmaceutical Research and Manufacturers of America *(2023) Global Access to New Medicines Report*.

What we heard:

> 'In theory, reimbursed access can be achieved within approximately 60 days of TGA registration if: there is parallel processing; a first time PBAC recommendation; and no delays to post-PBAC negotiations. In practice, however, there are very few cases where this is achieved.'
>
> **Consultation 1 submission: Medicines Australia**

Time to subsidised access was a key theme in Consultation 1, with several stakeholders acknowledging the disparity between what is technically possible and the reality.

The time it takes a new medicine on the PBS and make it accessible to Australians depends on several key steps by different decision-makers:

1. the date the pharmaceutical company launches the new medicine globally/in the first country
2. the time from global first launch to the pharmaceutical company applying to register the drug on the ARTG (and the PBAC if it is applying under parallel processing)
3. the time it takes for the TGA to review and approve the drug for the ARTG
4. the time it takes for the company to apply to the PBAC for listing on the PBS
5. the time it takes the PBAC to make a positive recommendation on the medicine
6. the time it takes to list the medicine on the PBS after it receives a positive recommendation from the PBAC.

The pharmaceutical company's global launch strategy determines the time and location for the global first launch of a new medicine. These strategies are commercial decisions that consider the return on investment and potential for profit maximisation from the medicine. Research shows that, generally, pharmaceutical companies launch their medicine in the US first and in other countries about a year later. This is likely because of the desire to launch first in a country with a large population base and the freedom to set the price before moving to other countries that use external reference pricing.[45] Australia has a relatively small population base; additionally, the price paid in Australia is used by other countries for external reference pricing.[46] Consequently,

[45] US Department of Health and Human Services (2024) *Comparing New Prescription Drug Availability and Launch Timing in the United States and Other OECD Countries [PDF 1226 KB]*.

[46] Zhang W, Guh D, Grootendorst P, Hollis A and Anis A (2024) 'The impact of changing the reference countries on the list prices for patented medicines in Canada: A policy analysis', *Health Policy*, 144:105064, doi: https://doi.org/10.1016/j.healthpol.2024.105064.

pharmaceutical companies' launch strategy often involve a delay in bringing medicines to Australia until the larger markets have been secured.[47]

When a sponsor decides to bring their medicine to the Australian market, they need to apply to the TGA to register it on the ARTG before it can be supplied. The TGA target time frame for processing applications for prescription medicines is 220 working days for the standard pathway and 150 working days for the priority review pathway. This equates to around 44 weeks and 30 weeks, respectively.

Technically, sponsors can make a submission for PBS listing as soon as they have applied for ARTG registration. The TGA and the PBAC applications will be progressed in parallel; however, a recommendation for listing on the PBS will not be made until the TGA application has sufficiently progressed, such that a positive TGA delegate's overview is available.

Despite parallel TGA and PBAC processing being available to all category 1 and 2 submissions, this is an underused pathway. Stakeholders reported that this is due to the PBAC being unable to provide a recommendation without a TGA delegate's overview. As a result, sponsors wait to apply to the PBAC to align the timing of the TGA delegate's overview with the PBAC meeting deadline.

What we heard:

> The parallel process works well but could be even more effective in reducing time to access by, for example, removing the requirement for a TGA delegate overview at the time of a PBAC decision.
>
> **Consultation 1 submission: Antengene**

Delaying the PBAC submission to align it with receiving the TGA delegate's overview is problematic for two reasons. The first is that the TGA time frames may not be consistent and a delay in receiving a delegate's overview results in the PBAC deferring the submission. The second issue arises when the PBAC does not recommend a medicine for listing on the first submission. Depending on which resubmission pathway the subsequent submission will go down, this means that the next meeting the submission can go to could be 34 weeks later.

[47] Incze A, Kalo Z, Espin J, Kiss E, Kessabi S and Garrison L (2022) 'Assessing the Consequences of External Reference Pricing for Global Access to Medicines and Innovation: Economic Analysis and Policy Implications', *Frontiers in Pharmacology*, 13:815029, doi: 10.3389/fphar.2022.815029.

The research and stakeholder input to the Review highlighted that most medicines that claim to have high added therapeutic value (HATV) that apply for PBS listing eventually get recommended. However, the first submission for new medicines is frequently not acceptable for the PBAC to make a positive recommendation.

In Consultation 1, stakeholders commented widely on the number of PBAC submissions required before a medicine receives a positive recommendation. Feedback indicated that some sponsors appeared to use the first submissions to the PBAC for early advice, rather than a submission with an expected successful recommendation.

Underscoring the two-submission system that appears to be standard in Australian HTA is, at least in part, due to the relatively short 17-week cycle. This short time frame represents a low opportunity cost for sponsors to obtain high-quality PBAC advice, compared to much longer decision cycles in other countries. The quality advice received from the first PBAC submission assists the sponsor to submit the more plausible scenarios and prices in subsequent submissions.

In Consultation 2, the Review tested options with stakeholders for reforms to reduce the time to access for new therapies with HATV in areas of HUCN, including strategies to:

- incentivise companies to bring their medicines to Australia earlier
- bring the first submission for a new medicine to the PBAC as early as possible
- minimise the time between the PBAC's initial and subsequent considerations
- maximise the chances that submissions will succeed within two considerations.

Conclusion

With the minimal use of parallel processing, the TGA and PBAC processing times are largely sequential. Coupled with the findings that many major new therapeutic advances require two (or more) submissions to an HTA committee before receiving a positive recommendation, the time from application for regulatory approval (ARTG registration) to positive HTA recommendation can typically be up to 95 weeks (44 weeks for a TGA evaluation plus 51 weeks for two submissions to the PBAC). This can be more protracted if more than one resubmission is required, or a longer period between the first and subsequent submission is taken. Additionally, this is further compounded by the delay in companies applying to the TGA relative to other countries with similar health systems and HTA arrangements (estimated median of 17 months).

Australia has exceptionally comprehensive health and medicines access systems, underpinned by high-quality HTA arrangements. However, compared to other jurisdictions like the United Kingdom and Canada, Australia has a small population base. To maintain comprehensive medicines access and the same quality of HTA advice to decision-makers, the Australian system needs to be able to process the same amount of HTAs as much larger countries, but with a relatively limited pool of HTA

professionals. Strategies to bolster the capacity and capability of the HTA system are further elaborated in Chapter 9. These will be supported by mechanisms to ensure the HTA processes are efficient and agile. Analysis and stakeholder inputs identified that, unlike many other countries, Australia does not have a system in place to prioritise submissions. In Consultation 2, options for reform included methods for differentiating the amount of effort pharmaceutical companies and HTA consultants spend in developing submissions, and the Australian Government spends assessing these submissions. These options were widely supported by stakeholders across different stakeholder groups. Aligning the HTA pathways and processes to ensure the HTA system expends time and effort where it is needed most will help maintain and improve timely, equitable and comprehensive medicines access.

Objectives of recommendations

The Recommendations in this chapter are intended to:

- reduce the time and effort that sponsors, the Department, evaluators and the PBAC spend on low-risk, simple submissions for therapies with no additional therapeutic advantage over existing alternatives
- ensure that the funding and assessment mechanisms and levels are proportionate to the complexity, risk and potential benefit related to the submission
- reduce the time to access for therapies with HATV.

Recommendations

Recommendation 7. Streamlined pathway for submissions using cost-minimisation analysis

The Review recommends:

a. the Australian Government, in consultation with stakeholders, develop and implement a streamlined appraisal pathway for submissions using cost-minimisation analysis. This pathway should include:
   i. developing criteria for therapies to be eligible for a streamlined pathway for submissions using cost-minimisation analysis
   ii. using an abbreviated evaluation for submissions for therapies that meet the criteria, and fast-tracking them to the price agreement stage after consideration by the Pharmaceutical Benefits Advisory Committee (PBAC)/PBAC Executive (or similar)
   iii. for submissions that do not meet the developed criteria, granting the PBAC Executive the ability to nominate for the submission to be considered without change by the PBAC or in the next cycle, to allow the sponsor time to address issues raised, noting the sponsor would have the discretion to withdraw their submission.

b. industry examines the feasibility and constraints, in consultation with relevant stakeholders, of sharing information about the effective price of the comparator for cost-minimisation submissions with the applicant sponsor before the HTA advisory committee considers it.

Recommendation 8. Improve the pathways and processes for listing therapies with high added therapeutic value in areas of unmet clinical need on the Pharmaceutical Benefits Scheme

The Review recommends:

a. the Australian Government, in consultation with stakeholders, enhance or replace the early resolution and/or facilitated resolution pathway with a more flexible pathway. The new pathway should support sponsors of therapies with high added therapeutic value in areas of high unmet clinical (HUCN) need to improve the quality of their resubmissions. The HTA advisory committee should also be given more time to consider submissions. This pathway should be proportionate, and the level of facilitation should be commensurate with the level of complexity and added therapeutic value of the submission. Enhancements should include:
    i. greater flexibility in the amount of time for resubmission than the current maximum of 19 weeks for the facilitated and/or early resolution pathways, with the amount of time determined by the HTA advisory committee
    ii. a resubmission timeline, where needed, to allow time for the Department of Health and Aged Care to:
        1. review the information in the resubmission to determine if and how the issues raised by the Pharmaceutical Benefits Advisory Committee (PBAC) have been addressed
        2. clarify outstanding issues with the sponsor, including whether the PBAC advice has been adequately addressed
        3. support the PBAC if it has questions or requires additional information.
    iii. facilitate these tasks by:
        1. assigning a case manager for the application
        2. providing additional commercial negotiation and economic modelling resources to work with the sponsor to resolve matters and present a comprehensive dataset to the PBAC to enable the committee to make decisions with the greatest degree of confidence
        3. the ability for the PBAC to hold stakeholder meetings with key patient and clinical organisations. The purpose of these meetings would be to make issues transparent to stakeholders, including making difficult trade-offs between the seller and buyer, transparent to other stakeholders.

Comment by Ms Elizabeth de Somer, Member Nominated by Medicines Australia: 'The industry recognises the need to ensure no perverse incentives are introduced into these pathways and recommends establishing independent dispute resolution and commercial negotiation processes.'

b. resource case managers to facilitate communication and information sharing between the Department and applicant for health technologies with likely high added therapeutic value in areas of HUCN and are using a cost-utility analysis or cost effectiveness analysis in their submissions. Consistent with other recommendations, the case management approach should be:
    i. proportionate to the level of complexity and potential clinical benefit of the therapy
    ii. developed in consultation with relevant stakeholders
    iii. consistent with best practice approaches across government and, if relevant, international counterparts
    iv. developed with clear and transparent roles and responsibilities, and principles governing interactions
    v. reviewed after 2 years against key performance indicators and the approach adjusted accordingly.
c. enable full parallel processing of Therapeutic Goods Administration (TGA) and PBAC submissions. It should do this by updating the PBAC Guidelines to enable the PBAC to communicate its likely advice to sponsors before receiving the TGA delegate's overview. The PBAC's final advice to the Government, the PBS listing, and resulting funding arrangements, would still need to be consistent with the Australian Register of Therapeutic Goods (ARTG) listing. (Note: this recommendation is not intended to limit the execution of 'Recommendation 2. Creating equitable access to medicines for children and young people').

Recommendation 9. Therapies with added therapeutic value

The Review recommends that the Australian Government, after a trial period and review, extend the mechanisms covered in 'Recommendation 8. Improve the pathways and processes for listing therapies with high added therapeutic value in areas of unmet clinical need on the Pharmaceutical Benefits Scheme', to all therapies claiming clinical benefit over existing alternatives. In line with 'Recommendation 3. Overarching recommendations for all HTA funding and assessment pathways and processes', this should follow a proportional approach.

Recommendation 10. Alternative modelling and analysis types for disease areas

The Review recommends that the Australian Government, in consultation with industry and other relevant stakeholders, investigate the feasibility and potential place for alternative types of analysis and modelling for disease areas. These should include:

a. a disease-specific common model
b. reference case modelling
c. whole-care pathway modelling that would enable the evaluation of the cost-effectiveness of different diagnostics and therapies across the care pathway.

The feasibility testing should include:

a. the potential for international collaboration in the development of models
b. consultation with industry and other stakeholders
c. the role of horizon scanning.

Why this matters

Following the recommendations in Chapter 4.2 would reduce the time frame for Australians to gain subsidised access to HATV therapies by around 16 months.

Typically, under the current system, a sponsor:

- does not apply for ARTG registration until an average of 17 months after receiving its first major overseas registration
- waits to receive ARTG registration before applying for PBAC consideration, rather than using parallel TGA and PBAC processing
- does not use one of the current early resolution or facilitated resolution PBAC pathways
- does not need more than one resubmission.

Note: Currently, the PBAC assesses resubmissions of HATV via the early resolution or facilitated resolution pathway, wherever possible.

Chapter 4.3: Application pathway for having vaccines listed on the National Immunisation Program

Introduction and context

The NIP provides free vaccines to eligible people to help reduce preventable diseases. It consists of a schedule (the NIP Schedule) of recommended vaccines by age group and/or medical risk. Vaccines on the NIP are free to Australians in the recommended age groups and risk groups.

As with other international jurisdictions, Australia uses a National Immunisation Technical Advisory Group to evaluate vaccines. The Australian Technical Advisory

Group on Immunisation (ATAGI) provides a wide range of advice for government and the public, including contributing to public health recommendations on the use of vaccines.

However, unlike many other jurisdictions, recommendations for the inclusion of vaccines in the NIP do not come directly from ATAGI, and they must be reviewed by the PBAC. To be considered for listing on the NIP, pharmaceutical companies must seek ATAGI advice before making a PBAC submission.

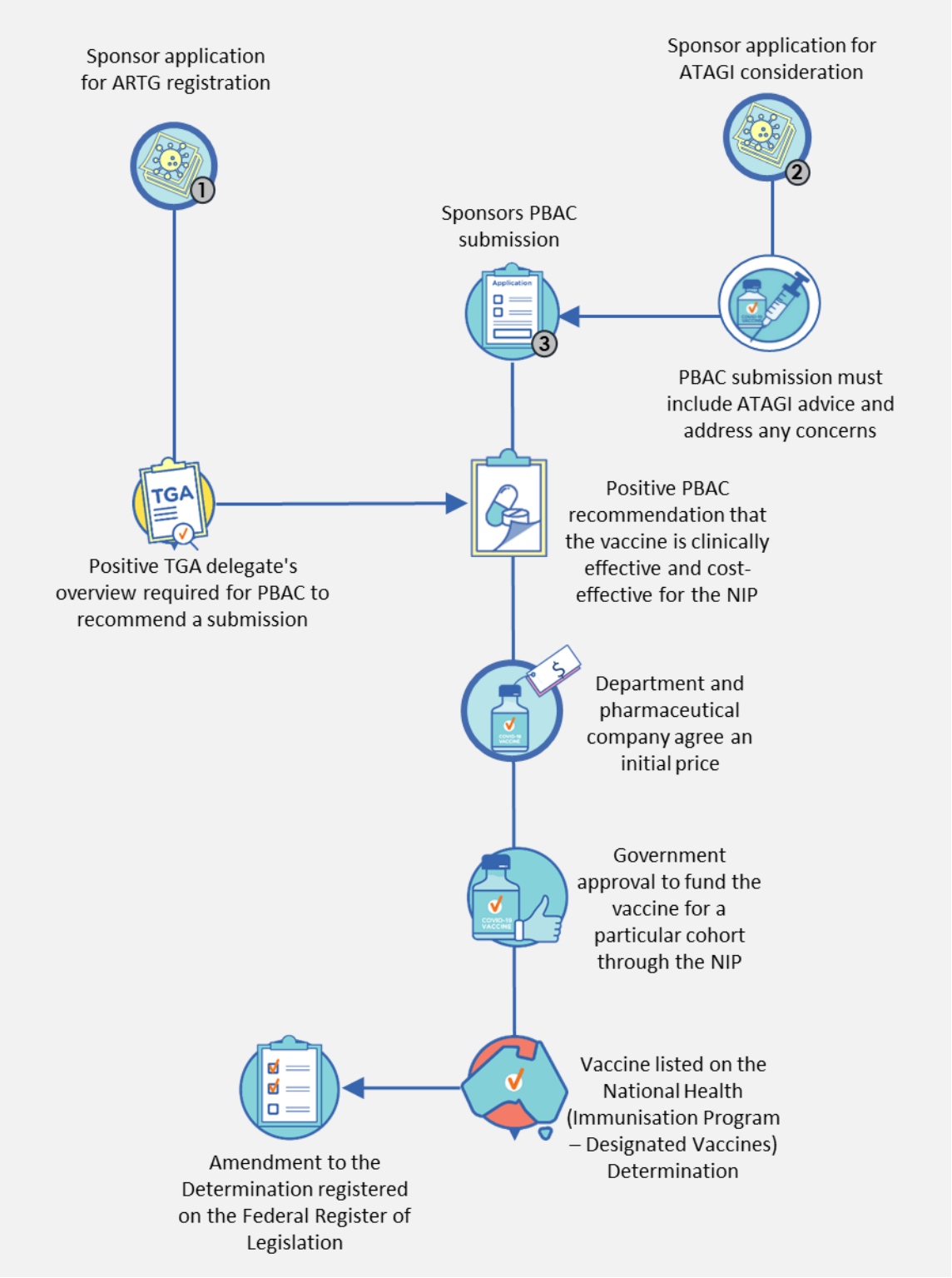

Figure 1: HTA pathway for vaccines being listed on the NIP

Findings

The minimum time for a vaccine to receive a positive HTA recommendation is 48 weeks, as the ATAGI process starts 31 weeks before the PBAC 17-week cycle.

The Review's early consultation relating to ATAGI highlighted the extended time frame for a vaccine to be listed on the NIP relative to the minimum time frame for therapies

to be listed on the PBS. Stakeholder comments indicated perceptions of potential duplication between ATAGI and the PBAC. Additionally, stakeholder sentiment indicated that the ATAGI processes were protracted, suggesting that it was perhaps due to the size of ATAGI and the difficulty of coordinating the full membership.

What we heard:

> 'The current vaccine assessment process is slow and cumbersome, with the average time taken to get TGA approval more than 3 years. There is a clear need in Australia to accelerate the process for introducing new vaccines on to the National Immunisation Program; the 2-step approval process, being ATAGI, then PBAC, is unusual. It would be useful to look at other models around the world where vaccine recommendation processes are much faster.'
>
> **Consultation 1 submission: Immunisation Coalition**

During Consultation 2, the Review tested a reform option with stakeholders to reduce the time needed for HTA processes before vaccines can be entered in the NIP. The option proposed streamlining the HTA pathway by allowing the sponsors of vaccines to apply to the PBAC to list a vaccine on the NIP without first applying to ATAGI. The submission would be evaluated and the PBAC ESC would be supplemented with appropriate representatives from ATAGI to provide joint ATAGI and PBAC ESC advice to the PBAC. Additionally, it was proposed to support this process by bolstering ATAGI's horizon scanning and allowing a more proactive approach to submissions.

Industry stakeholders were generally positive about the proposed reform, as long as ATAGI remained accessible and flexible. Clinical and scientific stakeholders were optimistic that it could reduce the time needed to get vaccines on the NIP, as long as the same evaluator group was used for the PBAC and ATAGI. ATAGI's feedback on mechanisms having a more proactive and proportionate response to submissions was positive; however, it expressed concern about the potential requirement to remove full committee advice to the PBAC in relation to the NIP.

ATAGI has 15 voting members with expertise in a range of areas, including infectious diseases, epidemiology, disease modelling, population health, general practice, paediatrics, maternal health, immunocompromising conditions, program implementation and nurse practitioners, as well as consumer representatives. Unlike other medicines, vaccines on the NIP are administered as a program at a population level to people who are predominantly well. This requires additional considerations, including the population-level effects of vaccines within a complex ecological system.

Further system analysis and stakeholder consultations identified opportunities to streamline the vaccine pathway, while retaining full ATAGI consideration, as required.

These include a range of measures to better integrate and harmonise the PBAC and ATAGI processes inside the Department and during evaluation. Improving alignment between the two processes would require additional resourcing to enable the ATAGI Secretariat to hire technical experts, similar to the PBAC Secretariat. Improved internal alignment combined with expanding the evaluator groups for PBAC submissions to include vaccine evaluation experts, would condense the time period leading up to ATAGI consideration. Additionally, it was identified that as ATAGI consideration takes place considerably earlier than PBAC consideration, updated data is often available by the time the PBAC considers the vaccine. This can cause process issues and sometimes delays. Bringing the ATAGI consideration of the vaccine closer to the PBAC consideration would mitigate this issue.

Additionally, more opportunities for process improvements and timeliness are available. These include allowing ATAGI to (further) differentiate its submissions and align the time and effort spent on each submission with the degree of complexity and risk. This would increase the time available for ATAGI and evaluators to consider more complex submissions and reduce timelines.

Research indicated that unlike many other countries, ATAGI and the PBAC rely on the economic model provided by the sponsor to inform their decisions about vaccines. Internationally, significant benefits are reported from using a more proactive approach, such as considering how new products or potential changes to vaccine programs would affect disease burden, followed by issuing invitations for submissions or tenders from relevant sponsors. ATAGI noted during consultations that sponsors' early provision (in confidence) of data, including updated or new data provided to the PBAC after ATAGI's pre-submission advice, is helpful as it enabled ATAGI to prepare high-quality advice.

Conclusion

ATAGI's processes add approximately 31 weeks to the PBAC HTA pathway (the 17-week cycle), resulting in approximately a 48-week HTA pathway for vaccines for NIP listing. By improving alignment between the PBAC and ATAGI pathways, processes and secretariat functions, and using the same evaluator group for both, the time to HTA recommendation would be reduced by 18–22 weeks, or around 40%.

This could be supported by proactive and proportional restructuring to maximise the chances of a positive HTA recommendation in the first submission, and further expedite access to crucial medicines for Australians.

Objectives of recommendations

The recommendations in this chapter aim to:

- make health technology funding and assessment methods proportionate to the level of risk and potential benefit of each therapy, ensuring time and effort in the HTA system goes where it is most beneficial

- improve time to access for new vaccines in areas of unmet clinical need
- support ATAGI to improve Australia's health security, with a proactive, agile and forward-looking system.

Recommendations

Recommendation 11. Proportionate appraisal pathways to align the Australian Technical Advisory Group on Immunisation assessments with the level of risk and complexity of the product

The Review recommends that the Australian Government:

a. support and resource the development of a framework to assess vaccine submissions for the level of risk and complexity of advice required, building on the advice provided for sponsors through the Australian Technical Advisory Group on Immunisation (ATAGI) Guidelines. This framework should be developed in consultation with stakeholders, including ATAGI, the Pharmaceutical Benefits Advisory Committee (PBAC), and academic experts and evaluators
b. develop a proportionate appraisal mechanism and pathway for vaccine submissions in consultation with ATAGI, the Therapeutic Goods Administration (TGA), the PBAC and industry that:
    i. has a single front door mechanism for vaccine sponsors to make submissions for the National Immunisation Program (NIP) to the TGA, ATAGI and the PBAC
    ii. includes resourcing to enable early triaging and preliminary evaluation of vaccine submissions to determine the complexity and risk of each vaccine submitted (based on the sponsor's self-nomination)
    iii. ensures the level of assessment effort is proportionate to the level of risk and complexity of the submission
    iv. is supported by the expansion of the evaluator groups used for the PBAC submissions to include vaccine evaluation experts to assess each sponsor's submission and produce a single comprehensive assessment report (reducing the time needed and duplication, and adding consistency and continuity)
    v. aligns ATAGI and PBAC Secretariat processes and functions
    vi. includes a process that allows for the ATAGI meeting to occur before the PBAC Economic Sub-Committee (PBAC ESC) meeting to enable the PBAC ESC to consider ATAGI advice.
c. examine the opportunity to delegate to ATAGI the authority to recommend a new vaccine for a disease if the vaccine is being cost-minimised to an existing PBAC-recommended vaccine.

Recommendation 12. Proactive vaccine assessment pathway

The Review recommends that the Australian Government develop a process for proactive modelling and considering how new products or potential changes to the vaccine program could impact disease burden and inform which vaccines, invitations for submissions or tenders are made for. This process should:

a. be developed in collaboration with the Australian Technical Advisory Group on Immunisation (ATAGI) and other relevant stakeholders
b. include the ability to undertake independent modelling as recommended by ATAGI
c. be supported by the development of a coordinated horizon scanning process including ATAGI, the Therapeutic Goods Administration (TGA), the Pharmaceutical Benefits Advisory Committee (PBAC), the Department of Health and Aged Care and the Australian Centre for Disease Control
d. include a process for early alignment of the Population, Intervention, Comparator, and Outcome – or PICO – scoping criteria
e. include requesting that sponsors engage early to discuss their vaccine proposals and provide relevant data to ATAGI as it becomes available, to ensure that ATAGI can prepare to provide advice.

Chapter 4.4: Pathways for highly specialised therapies and other therapies co-funded between the Australian and state and territory governments

Introduction and context

Many terms are used to describe therapies such as (gene-modified) cell and gene therapies. Internationally, the term 'advanced therapy medicinal products (ATMPs)' is used to describe medicines based on genes, tissues or cells that are used to treat often very rare and severe disease or conditions in many countries.

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 also use the term 'highly specialised technologies'[48] (including in some of the research papers commissioned for the Review) to refer to these

[48] Highly specialised technologies are similar to, but not the same as, highly specialised therapies, as defined in the 2020–25 Addendum to the National Health Reform Agreement.

therapies. Throughout this document, these therapies will be referred to as 'advanced therapies (ATs)', unless context requires the use of other terms.

The term 'High-Cost Highly Specialised Technologies' (HSTs) is defined in the 2020–25 Addendum to the National Health Reform Agreement (NHRA) (specifically for interpretation of the NHRA) as:

> 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 the 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.[49]

While the term HSTs used in the 2020–25 Addendum to the NHRA[50] predominately includes ATs, it can also include high-cost pharmaceuticals delivered to public hospital inpatients.

The approach to submission, evaluation and funding of advanced therapies has been an increasingly important matter raised by stakeholders in recent years. The majority of advanced therapies that are delivered in a public hospital setting are funded through a shared funding arrangement between the Australian and state and territory governments, as set out in the relevant clauses and schedules in the 2020–25 NHRA Addendum.[51]

Relevant to the Review's Terms of Reference are examinations of the governance and triaging processes that support submission, evaluation and funding of eligible advanced therapies. These are detailed in Appendix B of the 2020–25 NHRA Addendum.[52] In brief:

- Sponsors that consider their therapy meets the definition of an HST, as specified in the 2020–25 NHRA Addendum,[53] usually contact the Department to discuss the process for potential HSTs.

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[49] DHAC (2020) *2020–25 Addendum to the National Health Reform Agreement*.

[50] DHAC (2020) *2020–25 Addendum to the National Health Reform Agreement*.

[51] DHAC (2020) *2020–25 Addendum to the National Health Reform Agreement*.

[52] DHAC (2020) *2020–25 Addendum to the National Health Reform Agreement*.

[53] That is, TGA-approved medicines and biologicals delivered in public hospitals where the therapy and its conditions of use are recommended by the MSAC or the 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 the 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.

- Where the Department agrees that the therapy appears to meet the definition of an HST as outlined in the 2020–25 NHRA Addendum, the sponsor is required to provide a briefing to the jurisdictions on the therapy as a means of informing the jurisdictional representative on the Joint Chairs' Committee (including the MSAC Chair, the PBAC Chair and a nominated representative of the jurisdictions).
- The sponsor then lodges an MSAC application, facilitating consideration of the therapy by the Joint Chairs' Committee.
- If the Joint Chairs' Committee considers that:
  - the PBS is the most relevant funding program, the sponsor is informed of this and may proceed to submitting a PBAC application as per standard PBAC processes
  - the therapy is most appropriately funded under the 2020–25 NHRA Addendum,[54] the application proceeds to the MSAC for consideration, as per standard MSAC processes.

Findings

Availability of procedural information

It is crucial that key information on the submission, evaluation and provisioning steps for HSTs is available and visible, to give stakeholders appropriate levels of certainty and transparency.

Guidance available to sponsors in relation to whether the HST process outlined in the 2020–25 Addendum to the NHRA[55] applies to their advanced therapy (AT) products is lacking. This echoes observations in stakeholders' submissions to The New Frontier inquiry[56] and the Review. Apart from the Addendum itself, no publicly available guidance outlines the process.

Through consultation with the states and territories, the Review noted that the Health Technology and Genomics Collaboration is developing the *Framework for the assessment, funding and implementation of high cost, highly specialised therapies and services*. This will give stakeholders some additional visibility and clarity about the HTA processes for eligible ATs.

The Review also noted that the basis on which the Joint Chair's Committee determines the most appropriate funding mechanism for an HST is not well understood. Industry stakeholders in particular, queried whether the appropriate funding mechanism is

[54] Schedule C clauses C11 and C12, DHAC (2020) *2020–25 Addendum to the National Health Reform Agreement*.

[55] DHAC (2020) *2020–25 Addendum to the National Health Reform Agreement*.

[56] Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

primarily determined by defining the optimal patient care pathway, as opposed to whether a treatment could technically be funded on the PBS.

Once an HST is supported for funding by the MSAC, there is also a lack of publicly available information in relation to where and when access to HSTs funded via the 2020–25 NHRA Addendum arrangements becomes available. Additionally, information about patient referral pathways, eligibility criteria for access to HSTs, and associated processes relevant to the patient journey are unavailable.

Provisioning and implementation architecture

The recent experiences of evaluating, funding and provisioning chimeric antigen receptor T (CAR-T) cell therapies for cancer provide a useful case example, setting out some matters identified by stakeholders, and showing where potential reforms may be of most benefit.

Australian implementation experiences with CAR-T ATs revealed that when a novel therapy is introduced, appropriate time is required to enable health services to respond to the implementation requirements (including centre accreditation and service capacity). This is similar to implementation and site provisioning experiences internationally. The same ATs have required extended lead times to support capacity building and capability rollout in the health system.

However, participating Australian jurisdictions have advised that once treatment sites are equipped and have developed processes for delivering a particular type of AT, the time to implementing subsequent ATs that rely on the same services is significantly reduced. In these circumstances, the main factor impacting time to patient access becomes the time it takes for the sponsor to negotiate its Deed of Agreement for funding with the Commonwealth and supply arrangements with the jurisdictions.

The implementation experiences for CAR-T ATs have also highlighted the importance of high-quality post-HTA data collection and reassessment processes in the HTA context. To date, most applications considered for HSTs have been for subsidising ATs used in relatively small populations. These applications were usually supported by small single-arm trials, with a lack of long-term follow-up. Therefore, the magnitude of treatment effect and durability of response was uncertain at the time an initial application for public funding was considered, despite CAR-T ATs being referred to as a 'cure' for patients with significant long-term benefits.

The MSAC's subsequent review of tisagenlecleucel (Kymriah) for treating children and young adults with acute lymphoblastic leukaemia (ALL) in July 2023 showed that the treatment has been adopted in Australian practice as a bridge to transplant for a substantial number of patients. Additionally, a substantial number of patients continue to have progression of disease following treatment with the therapy (i.e. the majority

of patients have not reported the claimed long-term health outcomes).[57] While some patients remain disease-free, it remains unclear why some patients respond better to therapy than others (i.e. clinical uncertainties still exist). This disparity of health outcomes highlights the need for ongoing review of novel therapies, underpinned by complete and high-quality outcomes data (e.g. via a clinical registry) to allow the appropriate comparative analysis to be done.

Conclusion

Funding pathways for HSTs require further development in a number of areas. Australian and state and territory governments could be better prepared to provide access to these technologies through more coordinated horizon scanning. Implementation steps are not transparent and arrangements set up to manage the higher uncertainty associated with the safety, effectiveness, use and cost of these therapies have not been effective.

Objectives of recommendations

The recommendations in this chapter aim to improve processes, accountability and timeliness for HSTs and other therapies co-funded between the Australian and state and territory governments.

Recommendations

Recommendation 13. Improved processes, accountability and timeliness for highly specialised therapies and other therapies co-funded between the Australian and state and territory governments

The Review recommends that the Australian Government:

a. encourage and provide support for expediting the development and implementation of a nationally cohesive approach to HTAs as outlined in Schedule C of the 2020–25 Addendum to the National Health Reform Agreement (NHRA)
b. develop a national HTA framework, including processes for HTAs to inform advice on implementation, investment and disinvestment opportunities at national and state levels. This work should leverage work already underway through the Health Technology and Genomics Collaboration and align with the unified pathway (see 'Recommendation 4. Unified HTA pathway and committee approach for all Australian government funding of health technologies') where appropriate. The Australian Government should:

[57] See DHAC (2023) *MSAC Public Summary Document Application No. 1748 - Review of Tisagenlecleucel for treatment of confirmed relapsed/refractory CD19-positive acute lymphoblastic leukaemia in children and young adults up to 25 years old, July 2023 MSAC Meeting*.

i. develop and implement a methodology, in consultation with stakeholders, to consider the cumulative impact of high-cost, highly specialised therapies (HSTs) on the health system, at a national level. This should be used to inform the HTA decision and reviews of subsidised therapies that ensure the publicly funded therapies being delivered to patients represent the most appropriate treatment pathway for patients
ii. develop, in consultation with relevant stakeholders, criteria to ensure a nationally consistent process for patient selection and allocation for HSTs.

c. establish time frames for implementing HSTs, funded through the 2020–25 Addendum to the NHRA, where the therapy has a positive HTA recommendation. This should be modelled on targets agreed with respect to the time frames for listing medicines on the Pharmaceutical Benefits Scheme (PBS) (see 'Recommendation 15. Jointly owned performance targets). This process should include:
    i. within 3 months of reaching the in-principle pricing agreement, a national-level implementation plan being published in collaboration with state and territory governments. The plan should include timelines for implementation in the different jurisdictions, and details of how the state and territory governments will ensure their populations (consistent with the HTA recommendation) can access the treatment within 6 months of the in-principle pricing agreement
    ii. an overarching stakeholder explanation of the process following an HTA recommendation, including the reasonable time frames and responsible party for each step (in line with 'Recommendation 3. Overarching recommendations for all health technology assessment funding and assessment pathways and processes'. This information should be developed in consultation with stakeholders
    iii. publishing implementation progress information to improve transparency and accountability for all responsible parties (the Australian Government, the sponsor, and state and territory governments), including clearly highlighting causes of delays relative to the developed 'reasonable time frame' for any steps
    iv. the original Deed of Agreement to incorporate the requirement for the technology to be reviewed for clinical effectiveness and cost-effectiveness at an agreed period (this will assist with timeliness as it reduces the initial risk, allowing more flexibility).
d. work with state and territory governments and industry to establish (or participate in an existing international collaboration) a horizon scanning process (consistent with the principles for horizon scanning in Chapter 9.2) to identify, prioritise, assess and monitor high-cost HSTs funded through the 2020–25 NHRA Addendum. This process will ensure jurisdictions can begin early implementation planning for HSTs. Additionally:

i. The horizon scanning should include input from a broad range of stakeholders including patients or patient organisations, industry, academia and the research sector, and state and territory governments
ii. The horizon scanning should have performance measures to ensure it is efficient and effective for its purpose, and a mechanism for accountability of results to be actioned in preparation for and to inform implementation planning
iii. Joint funding by the Australian and state and territory governments and industry should be explored.

e. develop a framework for systematic 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. This includes providing state and territory health departments with opportunities for consultation and collaboration on HTA decisions that will have a significant financial or operational impact on them (see Chapter 9.4).

Chapter 4.5: Life-saving drugs for patients with ultra-rare diseases (Life Saving Drugs Program)

Introduction and context

The Life Saving Drugs Program (LSDP) is a medicines funding program separate from the PBS that funds specific, life-saving treatments for patients with ultra-rare diseases. In Australia, ultra-rare is defined as 1 case per 50,000 people or fewer of the Australian population, or fewer than approximately 500 people in the Australian population.

For a medicine to be eligible for the LSDP, the PBAC must have considered it to be clinically effective, but not sufficiently cost-effective to list on the PBS. It must also meet LSDP eligibility criteria as assessed by the LSDP Expert Panel.

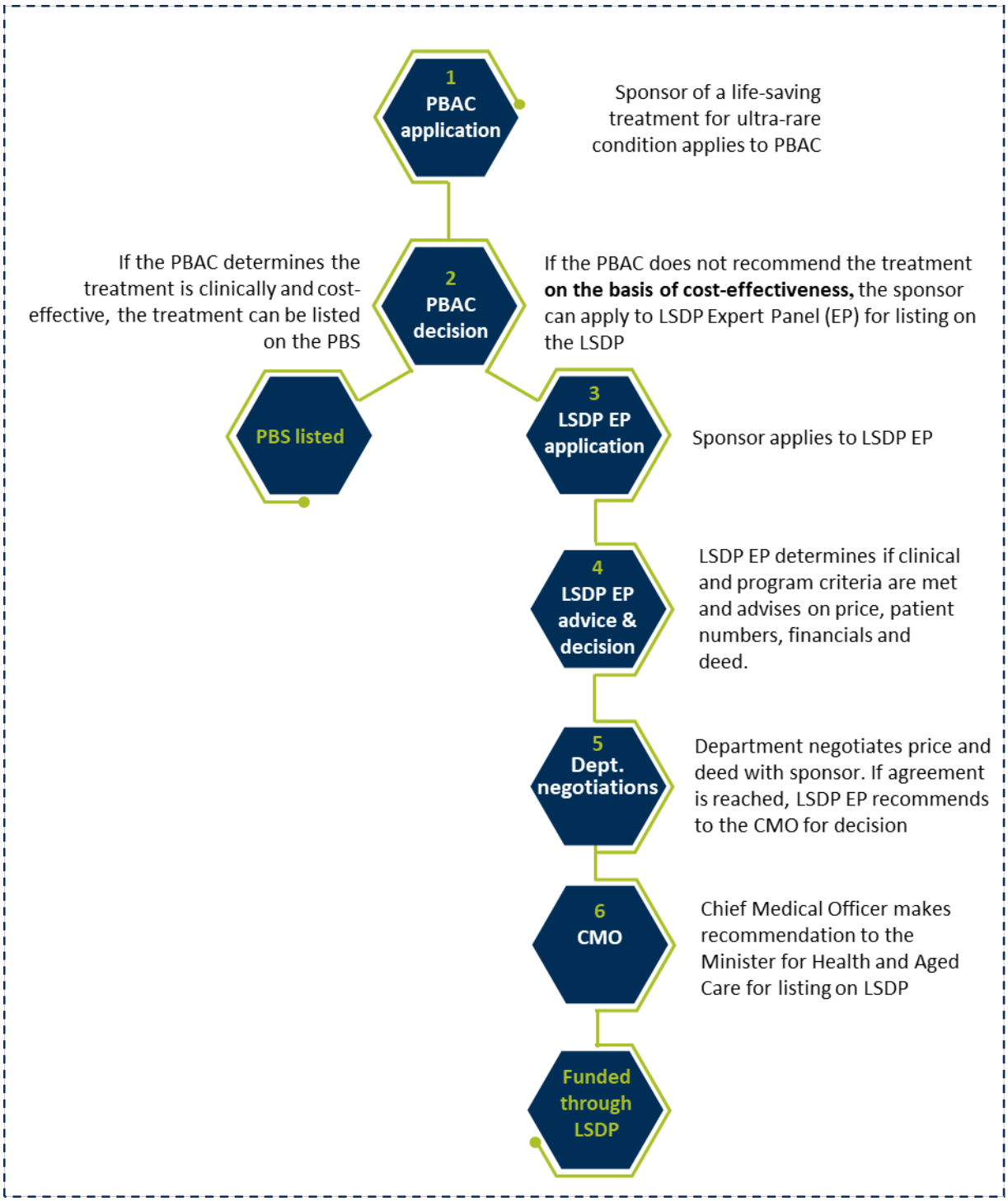

Figure 2: The HTA pathway for health technologies seeking funding through the LSDP

Findings

During the review process, the Review agreed that it was important to examine the prior experiences and current operation of the LSDP, to ensure HTA policy, processes and methods are well adapted to, and capable of, assessing health technologies that are best suited to provisioning and access through the LSDP arrangements.

Stakeholder feedback from patients, industry, service delivery and healthcare payers (i.e. governments) during consultations and as part of The New Frontier inquiry[58] consistently emphasised the importance of the LSDP as:

- an individual health technology access program for individual patients
- a broader example of a health policy approach that seeks to address health equity for under-represented sub-populations with ultra-rare diseases.

Stakeholder responses to proposed options for process reforms to streamline and align HTA pathways and advisory committees (including prospective realignment of LSDP HTA evaluation under the PBAC) were cautiously supportive and positive in-principle.

However, a subset of industry and expert stakeholders raised concerns regarding the possible consequences arising from the PBAC absorbing LSDP advisory functions under its program scope. These included possible changes in the way clinical and cost factors for health technologies would be considered as part of LSDP recommendations, and flow-on implications (positive and negative) for how LSDP recommendations would be translated into administrative processes that supported patient access to therapies. Both the option of continuation of stand-alone operations and the option of redesign into a Section 100 PBS program-style structure for administrative and program management efficiency[59] were both considered.

The Review also considered the additional observations from the LSDP Expert Panel about the importance of having a clear statement of rationale for the LSDP, consistent with the overarching recommendations of the LSDP medicines reviews in 2022.[60] In deliberating on this issue, the Review agreed that a clear statement of rationale would support transparency and stakeholder engagement by improving:

a. clarity about the principles underpinning the program, including the rationale for funding medicines for ultra-rare conditions, such as (but not limited to) health equity considerations
b. understanding of the cost implications associated with LSDP therapies, including research and development (R&D) for treatments, as well as costs to healthcare payers
c. engagement and dialogue about the interaction between eligibility and listing criteria, and broader medicines pricing and access policies (including clinical, cost and equity considerations), and the need to balance support for market

[58] Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

[59] See Alexion and LSDP Expert Panel submissions to Consultation 2 for different representative perspectives on these issues.

[60] DHAC (2022) *Life Saving Drugs Program (LSDP) Medicines Reviews Recommendations from the LSDP Expert Panel*.

authorisation and subsidisation, patient access and overall program sustainability.

Conclusion

The Review reiterates that any redesign of HTA pathways and associated policy and/or guidance for the LSDP needs to be sufficiently flexible and clear to stakeholders. Appropriate and flexible triaging, evaluation, recommendations and monitoring activities are essential to maintain alignment of the LSDP with the NMP objectives to ensure equitable, timely, safe and reliable access to medicines.

The Review also reiterates that the broad framework for developing advice and for decision-making to support listing of LSDP medicines remains sound. Recommended reforms continue to support the intended objectives of removing double-handling of submissions where possible, and supporting improved stakeholder understanding of the intent, purpose and operation of the LSDP (via a statement of rationale).

Objectives of recommendations

The proposed reforms discussed during deliberation are intended to provide:

- additional clarity and certainty on the essential purpose and intent of the program for participating stakeholders, including ensuring that appropriate eligibility criteria for the consideration and listing of therapies on the LSDP, and subsequent ongoing management arrangements, are clearly stated in relevant guidance and policy
- alignment with the broader observations the Review heard that double-handling and other administrative barriers should be eliminated where possible to support more timely access to important therapies for patients.

Recommendations

Recommendation 14. Improving time to access life-saving drugs for patients with ultra-rare diseases (Life Saving Drugs Program)

The Review recommends that the Australian Government:

a. develop and publish a statement of rationale for the Life Saving Drugs Program (LSDP) in consultation with stakeholders, including the LSDP Expert Panel. The statement should outline:
    i. principles underpinning the program
    ii. the eligibility criteria, including the value-for-money consideration, by reference to the overarching recommendations of the LSDP medicines reviews in 2022.
b. make necessary process and policy reforms (including updates to guidelines and stakeholder engagement materials) to enable:

i. the Pharmaceutical Benefits Advisory Committee (PBAC) (or its future functional equivalent) to become the sole HTA advisory committee for assessing and recommending funding of therapies for ultra-rare diseases
ii. the HTA advisory committee to source additional expertise and advice (including from entities such as advisory panels, patient communities and specialist clinicians) to inform and support recommendations regarding access to therapies for ultra-rare diseases
iii. the HTA advisory committee to advise the Minister on any requirements for subsiding a therapy for ultra-rare diseases, including evidence collection measures and disclosure of cost and efficacy information, consistent with the principles outlined in a statement of rationale for the LSDP (above).

Why this matters

Removing the extra steps in this pathway, and providing additional clarity and certainty for sponsors, will reduce the time for listing on the LSDP by around 4 months.

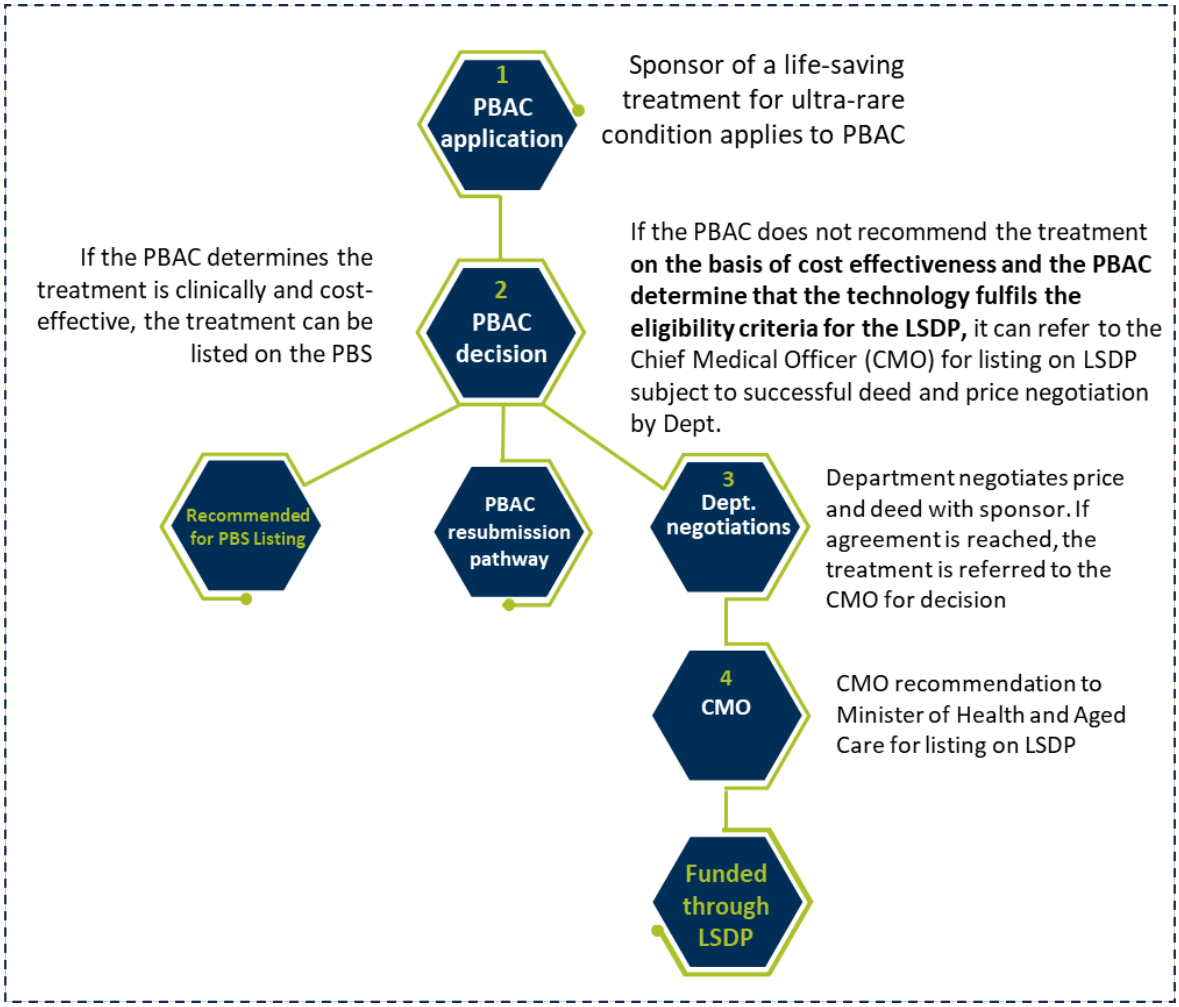

Figure 3: Proposed HTA pathway for health technologies seeking funding through the LSDP

Chapter 4.6: Measuring the impact of reforms on timeliness

Introduction and context

The Review was established in part to examine how to improve the timeliness of access to new therapies. Inputs to the Review from the House of Representatives' The New Frontier inquiry and submissions from many stakeholders indicated that this was a high-priority goal. Access to new therapies that deliver greater benefit than existing therapies, and those in areas of high and currently unmet need, was a particular focus. The inquiry noted that since most patients cannot afford the expense of many new medicines, they must wait until they are reimbursed by the Government. This wait time can significantly impact the lives of patients who need immediate treatment, and their families and carers.

Findings

Data on the time frames for approval of medicines contained in the paper *Australian market authorisation, funding and assessment pathways and timelines*[61] indicate the determinants of how promptly a therapy is made accessible in Australia compared to the US or Europe. These are 1) the decisions of the sponsor on the timing of a submission to the TGA for Australian Register of Therapeutic Goods (ARTG) registration and the timing of the submission to the PBAC or the MSAC for reimbursement, and 2) the duration of the HTA, including the time to reach agreement between the sponsor and the Government on pricing and conditions of supply.

For drugs recommended for listing on the PBS, there is a comparatively minimal delay in implementation (the standard time frame for Services Australia to update its payment system for PBS is generally 6 weeks for new PBS listings). However, for therapies funded through other measures (i.e. the MBS, joint Australian and state or territory agreements, or the LSDP), additional significant delays are apparent.

Measures to minimise the time to access require the seller (sponsor), the HTA bodies and the buyer (the Government) to commit to negotiating in good faith and with the clear intent of achieving agreement in an appropriate time frame. From patient and clinician perspectives, this time frame should be as short as possible for drugs that deliver major therapeutic advances (i.e. they demonstrate superiority). No element in the HTA process requires the sponsor or the Government to reach agreement in a time frame that is patient-centred.

In 2021 and 2022, submissions for new drugs demonstrating superiority required on average more than two considerations by the PBAC (range 2–4). Only 50% were listed on the PBS within 22 months of ARTG registration (i.e. TGA approval).

Conclusion

The recommendations in Chapter 4 and Chapter 5, if accepted, will improve timeliness of access.

Acceptance of the Review's recommendations should lead to sponsors achieving positive HTA recommendations after one or no more than two submissions for products demonstrating superiority. If parallel processing is used effectively, 90% of these products could be listed on the PBS within 6 months of ARTG registration. Without parallel processing, they could be listed within 12 months.

The Review considered whether the number of submissions for a given product for a given indication could or should be limited (e.g. to two). Feedback received through

[61] DHAC (2023) *Australian market authorisation, funding and assessment pathways and timelines*, Health Technology Assessment Policy and Methods Review.

Consultation 2 indicated that this would be too restrictive from an industry perspective and may introduce unintended negative consequences for timeliness.

The Review instead puts forward suggested targets that, if achieved, are significant advances in achieving timeliness for access to the great majority of products that demonstrate superiority. Complementing these targets, the Review recommends publishing information that enables identification of issues that require ongoing attention, to further improve timeliness.

Objectives of recommendations

The recommendations aim to assist the Government and industry to demonstrate to the Australian community their commitment to enabling timely access to new therapies. Public commitment to this in the form of agreed targets and performance metrics that are jointly owned and published will increase accountability, transparency and HTA system performance.

Recommendation

Recommendation 15. Jointly owned performance targets

The Review recommends that the Australian Government and industry reaffirm their commitment to good faith negotiations aimed at minimising the time to completing HTAs and commercial agreements for products claimed to be superior to existing care. They should also negotiate reciprocal commitments to these elements in any agreement, including agreed performance metrics that are compiled and published annually.

The Review recommends:

a. the introduction of reciprocal commitments including headline targets of:
    i. >90% for a Pharmaceutical Benefits Scheme (PBS) listing within 6 months of an Australian Register of Therapeutic Goods (ARTG) registration for registered products demonstrating superiority and submitted to the Pharmaceutical Benefits Advisory Committee (PBAC) in the first cycle following Therapeutic Goods Administration (TGA) submission under the TGA and PBAC parallel processing pathway
    ii. >90% for PBS listing within 12 months of ARTG registration for registered products demonstrating superiority, other than where (i) applies.
b. production of annual summary documents that transparently report instances where a product claimed to be superior is not PBS listed after two submissions, or not listed within the time frames specified in this recommendation (a (i)). For each product, the stage of assessment where progression to listing is primarily delayed should be specified in plain language, as well as the basis for this (where publicly available), using categories such as:

i. Stage – PBAC submission; Basis – parallel processing not used, PBAC application >3 months after ARTG registration, submission withdrawn
ii. Stage – PBAC consideration of claim of superiority; Basis – claim not accepted, claim acceptance required second consideration
iii. Stage – Commercial negotiation
iv. Stage – Implementation; Basis – guarantee of supply timing, Cabinet consideration of timing, high-level complexity in implementation.

c. that the impact of any accepted recommendations from the Review on the timeliness of access for therapies with proven superior clinical benefit be reviewed 2 years after implementation has commenced, and the results of that review are published. That review should consider:
   i. medicines submitted for listing on the PBS
   ii. medicines listed on the Life Saving Drugs Program (LSDP) (or its equivalent)
   iii. advanced therapies (agnostic of the HTA body; including highly specialised therapies (HSTs) funded through the 2020–25 Addendum to the National Health Reform Agreement (NHRA))
   iv. therapies, other than HSTs, submitted for consideration by the Medical Services Advisory Committee (MSAC).
d. that findings from the 2-year review should inform actions aimed at further improving timeliness to access to new therapies by removing unanticipated barriers that arise during or after implementation of any recommendations. The Review findings should also inform the revision of performance metrics and the negotiation of headline targets with reduced times to access.

Comment by Elizabeth de Somer, member nominated by Medicines Australia: 'The industry supports mutually agreed targets that reduce delays in patient access and recommends that a time frame for PBS listing within 60 days of ARTG registration for all submissions should be a future target.'

Chapter 5: Policies, methods and processes supporting the translation of HTA recommendations into patient access

Introduction

While a positive recommendation from an HTA advisory committee is an essential step in the overall process, it is not the end step that results in subsidised patient access to a health technology. Government-subsidised access to health technologies requires Australian Government agencies to work with stakeholders (including industry sponsors and other government agencies such as Services Australia) to translate the positive HTA recommendation into the essential legal, administrative and contractual materials that mean:

- patients can access the health technology at an affordable (subsidised) cost through established and familiar access channels (i.e. pharmacies or health service providers, depending on the health technology type)
- the sponsor and/or health service provider are paid
- the Government can defend to citizens its decision to subsidise access to the health technology as an effective and efficient use of limited resources to improve health outcomes.

This translation phase involves, at a broad descriptive level:

- negotiations on price and necessary conditions of access and availability
- a decision to fund by the Government
- listings processes to support administering a subsidy or reimbursement.

For some health technologies, this translation phase is complicated due to:

- the need for stakeholders to address and manage uncertainties (in the short term and in future years) about the overall benefit, harms and costs of the health technology identified during the HTA
- differences in negotiating positions and perspectives about price and access conditions
- stakeholder uncertainties about the next steps in the negotiation and listing process (including responsibilities and timelines).

These complications can add time to the translation step and slow down access to health technologies.

A guiding consideration of the Review was how it can advance the NMP's commitment to a system that is sufficiently flexible 'to rapidly respond to emerging and disruptive technologies. This includes innovative and highly specialised therapies and services, especially in circumstances where individuals have high unmet clinical needs. It also includes balancing equity, affordability and value-based healthcare objectives, and the quality use of medicines.[62]

The efficiency and effectiveness of post-HTA translation activities and processes are essential to advancing these NMP objectives. However, an efficient, equitable and sustainable health system underwritten by public subsidies also requires:

- having in place the right mix of activities and processes that support the important process of reassessment and re-examination of a health technology's place, price and value in clinical care throughout its lifecycle, and as new health technologies enter into use
- different approaches to funding and providing timely access to health technologies for patients in cases where the risks and benefits are more uncertain and may have significant costs attached.

This chapter covers:

- analysis and findings on the post-HTA arrangements that support how access to a health technology is negotiated, reimbursed and managed throughout its utilisation lifecycle in health care
- recommendations on these post-HTA arrangements that aim to improve the balance between access, cost, timeliness and quality of decision-making associated with health technologies, and facilitate improved health technology access for Australian patients, which are grouped into the following themes:
  - alternative ways to pay for health technologies (Chapter 5.1)
  - improving the post-HTA negotiation process (Chapter 5.2)
  - the need for regular reassessment of health technologies (Chapter 5.3)
  - practical approaches to managing uncertainty and risk, while supporting patient access to health technologies (Chapter 5.4)
- the combined application of some of the concepts described in this chapter for improving access to health technologies that address antimicrobial resistance (Chapter 5.5).

[62] Pillar 1 and Pillar 3, DHAC (2022) *National Medicines Policy 2022*.

Chapter 5.1: Alternative ways to pay for health technologies

Introduction and context

In recent years, as there have been advances in science and the price of many health technologies has risen, many health systems (including Australia's) have been faced with the practical problems associated with implementing HTA recommendations for comparatively cost-effective health technologies that have:

- a significant upfront and short-term budgetary (cash flow) impact on service providers and healthcare payers, and/or
- promising but uncertain quality and quantity of enduring health benefits due to the lack of long-term evidence.

Examples include:

- cell and gene therapies for which high, one-off upfront prices[63] may be payable for the health technology and treatment delivery to patients in clinical settings, but which have significant uncertainty regarding clinical benefit for patients in the long term
- health technologies that may present significant short- to medium-term budgetary pressures for healthcare payers due to demand; for instance:
    - where patient population eligibility is likely to be expansive and significant due to the population burden of the disease (e.g. new diabetes and obesity treatments)
    - where a latent patient population is awaiting subsidised access to a health technology that presents a major advance in treatment (e.g. curative Hepatitis C therapies).

These types of funding and financing problems are unlikely to be solved by the current standard approaches to paying for health technologies (i.e. 'price per unit' arrangements).

The Review sought to examine whether alternative approaches to health technology financing and purchasing may be necessary and relevant to the Australian healthcare context to address the increasing range of clinical, price and budgetary uncertainties that healthcare payers are seeing in practice. At the same time, these approaches would need to continue to meet patient and community expectations for equitable, timely,

[63] For example, the April 2024 PBS price for Zolgensma, a gene therapy to treat spinal muscular atrophy, is $2.527 million.

safe and reliable access to health technologies in a modern health system, consistent with the key pillars and principles of the NMP.[64]

Why this matters

If there are different ways for healthcare payers (i.e. governments) to finance and pay for health technologies, this could improve the availability of important treatments for patients while:

- better managing risks that the claimed benefits of health technologies are not reflected in patient health outcomes or do not provide value at the negotiated price
- minimising the opportunity costs of requiring large amounts of funding to be set aside upfront for health technologies that would take resources away from other expenditures
- minimising the need for additional taxes to fund the availability of health technologies in an otherwise competitive budget environment of finite Government resources.

Findings

Alternative financial payment models are being tested in different healthcare contexts to address the practical problems and risk associated with the use of high-cost/high-impact health technologies in health care. These included:

- specific patient-level product warranties (i.e. refunds for patients who do not respond to treatment)[65]
- annuity or mortgage payment programs (i.e. spread reimbursement for a health technology over multiple fixed payments and an extended time frame)[66]
- volume-delinked subscription-style bulk-funding program arrangements (i.e. providing an agreed fixed amount of funding to cover all access to a health technology over a defined period).[67]

The Review also noted that using alternative financial arrangements like these has improved negotiation outcomes. They have facilitated health technology access that may not otherwise have occurred due to concerns about upfront costs and/or possible inefficient use of healthcare resources if patients do not respond to treatments as expected.

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[64] DHAC (2022) *National Medicines Policy 2022*.

[65] Sanofi (n.d.) *Product warranty program in the USA for Cablivi® (caplacizumab-yhdp)*, Cablivi® website.

[66] See table 3 in Simoens S, De Groote K and Boersma C (2022) 'Critical Reflections on Reimbursement and Access of Advanced Therapies', *Frontiers in Pharmacology*, 13:771966, doi: 10.3389/fphar.2022.771966

[67] US Department of Health and Human Services (2020) *Eliminating Hepatitis C in Louisiana: An Innovative Payment and Outreach Model Case Study*.

Stakeholder perspectives on the use of these alternative tools and mechanisms to manage the budget impact implications of high-cost and high-impact health technologies were cautiously positive.[68] However, this support was tempered by observations that no specific alternative payment models were explicitly prescribed for immediate application into negotiation processes. Any use of such mechanisms should also facilitate balancing risk sharing with improving patient access to health technologies.

Conclusion

The Review reiterates its initial findings about the need for health technology financing and purchasing to be flexible and allow for different ways to improve the timeliness and availability of health technologies for patients, and to take a more balanced approach to risk and uncertainty. The Review suggests that in some cases, alternatives to the 'cost per unit' approach may be the most appropriate way to balance the needs of stakeholders by addressing health technology benefits and value considerations. At the same time, the Review also acknowledges that decisions to invest in subsidies for new health technologies are not made in isolation from the Government's broader healthcare and non-healthcare expenditure decisions.

The Review emphasises that all HTA stakeholders will need to be open to adopting different methods of paying for health technologies and embedding these approaches into workable contract and policy frameworks. This may require:

- scoping of payment- and financing-related issues between key parties before and/or during HTAs
- much earlier discussions about budget impact analysis compared to current arrangements
- better integration of these considerations into earlier phases of HTA processes to provide HTA advisory committees with additional details that may inform deliberations and recommendations.

Finally, if the Australian Government considers that these recommendations merit further exploration for implementation, appropriate additional consultation with stakeholders and other government agencies (principally the Department of Finance) would be required to identify and address additional practical implementation risks. These include how the respective parties' expenditures and revenues will be considered in the context of financial and accounting obligations.

[68] See Rare Voices Australia and AstraZeneca submissions to Consultation 2 for representative views of this general observation.

Objectives of recommendations

The Review's recommendations provide stakeholders with the opportunity to design and adopt new approaches to how health technologies are funded. This would ensure the approach to payment and financing does not become a limiting factor in improving patient access to health technologies.

Recommendations

Recommendation 16. Addressing the implications of high-cost/high-impact health technologies

The Review recommends that the Australian Government:

a. work with stakeholders (principally industry and government entities) on designing a framework that supports the use of different contract and health technology funding mechanisms, in addition to the standard 'price per unit' approach. These may include (but need not be limited to) mechanisms that facilitate more timely patient access to high-cost/high-impact health technologies, such as (but not limited to) mortgage-style regular payments, volume-delinked subscription-style reimbursements and/or patient-level product warranties
b. design the framework guided by the principles of:
    i. promoting earlier dialogue, design and negotiation of key parameters and expectations for financing and contracting parameters
    ii. facilitating the adoption of different funding and purchasing mechanisms that address the specific clinical, economic or budgetary issues and uncertainties that may be associated with the health technology in a risk-proportionate manner
    iii. maintaining consistency with other recommendations in this report that relate to pricing, subsidies and risk management–related matters.

Chapter 5.2: Improving the post-HTA negotiation process

Introduction and context

Following a positive HTA recommendation, a number of negotiation and administrative steps[69] need to be completed before a health technology can be provided as a subsidised treatment to patients. These can include (but are not limited to):

- negotiations on price

[69] DHAC (2020) *Procedures for a positive recommendation to list*, Pharmaceutical Benefits Scheme.

- agreement on the expected use and budget cost to the Government
- necessary restrictions on language that may apply to the health technology, consistent with the HTA advisory committee recommendation
- establishment of a Deed of Agreement and/or related documentation setting out any specific terms and conditions of supply, access and expenditure limits
- administrative processes necessary to ensure the health technology can be subsidised legally, consistent with the relevant legislation and/or regulations.

The time required to complete these steps varies, depending on the specific contextual issues for any given health technologies, but generally it takes less than 6 months.[70]

The New Frontier inquiry detailed 'stakeholder unhappiness' with several post-HTA decision processes. These included the length of the price negotiation process and the finalisation of PBS listing terms with sponsors,[71] and noted the importance of having these concerns examined by the Review.

By definition, the negotiation process can create a dynamic tension where:

- healthcare payers are incentivised to seek the lowest possible purchase price and minimise operational risk to meet legislative and social obligations for the efficient use of public funds
- 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
- patients cannot receive subsidised access to health technologies until the negotiation process is finalised.

This means that the length of the negotiation required to settle all relevant terms and conditions may result in an opportunity cost that is inefficient and does not yield the best balance of outcomes for all stakeholders. For individual patients and specific health technologies, delays in access may have profound negative consequences.

With this in mind, the Review sought to examine whether changes to post-HTA negotiation policies and processes could be recommended that would improve the timeliness and efficiency of decision-making and result in better patient access to health technologies.

[70] See table 12 in DHAC (2023) *Australian market authorisation, funding and assessment pathways and timelines*, Health Technology Assessment Policy and Methods Review.

[71] See cited evidence in Chapter 6 in Australian Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

Why this matters

Post-HTA negotiations and finalisation of administrative processes are key to translating a positive HTA recommendation into a Government-supported legal and administrative arrangement that provides subsidised patient access to a health technology.

While some health technologies proceed through negotiation and administrative steps very quickly, many others do not for reasons that are not always clear to stakeholders not involved in these processes.

Improving the way negotiations are conducted, and/or administrative arrangements finalised, could improve timely access to health technologies while ensuring efficient use of negotiation resources and time for all parties.

Findings

The Review noted that during deep-dive dialogue, those from outside the industry had a less comprehensive understanding of the key steps and time required to complete necessary activities to support subsidised access to a health technology after a positive HTA advisory committee recommendation is issued.

The Review also heard from industry stakeholders that key drivers of uncertainty and delay in health technology access are:

- the lack of certainty about how a positive HTA recommendation (including any incremental additional clinical benefits of a health technology) would be accounted for in pricing offers and inform negotiations between sponsors and healthcare payers
- the potential need to take into account any existing pricing policies for health technologies (such as approaches applied to some cost-minimised health technologies) when designing pricing offers and negotiating budget impact.

In examining international approaches to this issue, the Review noted examples where health systems have sought to provide more explicit guidance to health technology sponsors (or, in some cases, issued regulatory instruments) in respect of pricing expectations and boundaries. These were generally guided by the relative clinical benefit of a health technology (as identified in an HTA evaluation) to facilitate faster price-setting and negotiation processes. Examples 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 or value compared to existing treatments (such as the arrangements in place in Germany)

- tiered rebate or 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 and/or health equity significance (such as the arrangements in place in Japan).[72]

In testing a possible reform that would establish a pricing and negotiation guidance framework and directly link HTA recommendations to specific price negotiation parameters, stakeholders accepted the need for a pricing offer and negotiation guidance framework. Submissions generally supported more efficient and transparent post-HTA recommendation arrangements and stakeholder navigation of these arrangements. However, there was divergence within and across key stakeholder cohorts (patients, clinicians and industry) on:

- the matters that should be addressed through the frameworks, and how exchange of information and negotiation itself could be simplified or made more flexible
- the level of detail and prescriptiveness of the guidance framework, including any linkages between incremental health benefits and price negotiation parameters
- whether the framework would be in addition to, or replace the current pricing offer and negotiation framework, and whether it was necessary at all if other opportunities to engage in more flexible negotiation dialogue were provided
- associated stakeholder accountability for any reasons for delays in negotiations and resulting delays in subsidised health technology access for patients.[73]

Alternative concepts related to negotiation processes were put forward during the Review, including:

- introducing 'circuit-breaker'/third-party mediation contingencies (including resurrecting the former Pharmaceutical Benefits Pricing Authority) to break impasses between the principal negotiation parties and finalise negotiations within a defined period[74]
- providing different ways to share essential information earlier in the process of conducting an HTA, such as earlier disclosure of necessary reference price and/or Deed of Agreement information to sponsors (e.g. a comparator price under a special pricing arrangement for a cost-minimised health technology) to

[72] Further details of these examples are described in Adelaide Health Technology Assessment (2023) *International Health Technology Market Approval, Funding and Assessment Pathways, Health Technology Assessment Policy and Methods Review* within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

[73] See observations in the Consensus Letter from 51 consumer organisations, the AstraZeneca submission, the AbbVie submission and the Novartis submission to Consultation 2 for examples of diverse viewpoints.

[74] See Medicines Australia's submissions to Consultations 1 and 2 for representative views on this matter.

inform HTA submission drafting and/or decisions to withdraw submissions before a final HTA advisory committee recommendation.[75]

Elements of these alternative ideas may merit further consideration. These ideas are also intertwined with other pricing-related policies linked to the broader health and economic policy decision-making. However, these are beyond the scope of the Review, and/or would require a level of consultation and negotiation between principal parties (healthcare payers and individual industry stakeholders) that is beyond what the Review could do.

Additionally, overall stakeholder feedback suggested a general lack of appetite to progress these alternative ideas without further contextual knowledge of the broader package of reforms that might arise from this Review, and how these may in turn affect existing pricing-related policies (such as the statutory price reduction framework or policies around confidential pricing).

Conclusion

There are significant disparities in stakeholders' understanding of post-HTA processes, across stakeholder cohorts and within individual stakeholder cohorts. This suggests clearer public documentation is needed to explain the operation of pricing-related principles (such as the application of reference pricing rules) and the scope of activities required to support a subsidised listing following a positive HTA recommendation.

There was general stakeholder support for a more consistent, transparent and accountable approach to price negotiation arrangements, and the importance of such arrangements in providing the necessary predictability and visibility of post-HTA evaluation processes for non-negotiation parties (i.e. clinicians, patients and the general population).

However, this was tempered by a range of industry and consultancy stakeholder responses that were opposed to these ideas, either:

- on principle (as there should be no need to negotiate additional terms and conditions as all relevant information is within the positive HTA recommendation), or
- because of the risk of lost flexibility in settling important price and condition parameters if a specific framework had to be followed prescriptively.

The Review considered whether the divergent viewpoints on the need for, and scope of, a pricing and general negotiation framework represents the different levels of stakeholder understanding and success in navigating the post-HTA recommendation process. Improving the clarity, consistency and transparency of the negotiation

[75] This idea was discussed by the Reference Committee in response to information in Centre for Health Economics Research & Evaluation (2023) *HTA Methods: Economic evaluation*, Health Technology Assessment Policy and Methods Review.

pathway and process for all stakeholders is therefore an essential first step to removing some of the ambiguity surrounding the post-HTA process. This in turn should support improvements in the timeliness and success rate of translating HTA recommendations into subsidised health technology access for patients.

The Review encourages stakeholders to engage in ongoing dialogue to determine how additional details clarifying the operation of post-HTA negotiations and listing processes can be incorporated as systemic issues are identified over time. This would mean that any misunderstandings about the process or misapplications of supporting guidance can be addressed appropriately, and support stakeholder engagement with a consistent and predictable post-HTA process.

Objectives of recommendations

The Review's recommendations are intended to improve general and specific understanding of the pricing, negotiation and listings processes. Improved visibility and understanding of the processes and any linked policies that affect the way stakeholders engage with the negotiation processes are intended to improve the timeliness and success rate of translating HTA recommendations into subsidised access to health technologies for patients.

Recommendations

Recommendation 17. Pricing offer framework

The Review recommends that the Australian Government:

a. publish (after appropriate consultation and development) a post-HTA pricing, negotiation and listing policy framework (with associated supporting guidance documentation) that would apply to health technologies that have been positively recommended by the relevant HTA advisory committee
b. design and regularly update the framework to:
    i. provide stakeholders with necessary clarity where there are interactions with related pricing and health technology funding policies that need to be considered (e.g. pricing rules applicable to different forms or brands of medicines, or opportunities to discuss alternative contract and funding mechanisms)
    ii. improve visibility of the framework and associated supporting guidance documentation, to support better stakeholder engagement (including using existing modes of outreach and information sharing, such as the Medicines Status Website, Health Products Portal and the Pharmaceutical Benefits Scheme (PBS) website)
    iii. support future stakeholder engagement where changes to the framework may be necessary over time, including on matters such as (but not limited

to) transparency, timeliness, accountability and any necessary arbitration and/or mediation protocols to support finalisation of post-HTA processes.

c. provide any necessary resourcing requirements for expanded Commonwealth negotiation capacity and capability, in support of proportionate pathways reforms (see 'Recommendation 3. Overarching recommendations for all health technology assessment funding and assessment pathways and processes').

Chapter 5.3: The need for regular reassessment of health technologies

Introduction and context

The initial HTA evaluation is important to help stakeholders (principally healthcare payers) understand the clinical place and benefit of a health technology and make equitable and efficient decisions about allocating health resources. However, the initial HTA evaluation is only a single point-in-time examination of the evidence presented for a health technology.

As new health technologies are introduced into the healthcare marketplace, the clinical place and healthcare value of these previously evaluated health technologies will evolve, and the uptake and utilisation rates will change in response to new approaches in healthcare delivery. Follow-up monitoring may also reveal that some health technologies that showed promise in smaller populations and controlled settings do not improve health outcomes to the same degree when deployed more broadly, or in rare cases may contribute to patient harms (clinically and financially).

This can mean that after a period, the initial recommendations arising from the HTA evaluation may no longer be current, as the health technology may be displaced as best practice or rendered obsolete for the clinical indication in question. These circumstances in turn may justify the considered reallocation of finite health resources to improve and support overall patient health outcomes across the population.

In response to this evolving health lifecycle context for a health technology, countries that conduct HTA evaluations in support of healthcare payer reimbursement decisions for new health technologies also have a range of health technology review and/or disinvestment programs in place. The key purpose of these programs is to collect and evaluate current data on previously evaluated health technologies, and update recommendations relating to their quality use. This in turn may inform revisions to the appropriate efficient price for those health technologies.

In Australia, the primary programs supporting review of existing health technologies are the PBAC Drug Utilisation Sub-Committee (DUSC)[76] and the post-market review

[76] DHAC (2024) *Drug Utilisation Sub Committee (DUSC)*, PBS.

(PMR) arrangements,[77] which fall under the quality use of medicines objectives (pillar 3) in the NMP.[78]

DUSC outcome statements and utilisation analysis reports:

- assist stakeholders, including consumers, health professionals, researchers and pharmaceutical sponsors, to better understand how PBS medicines are being used, the methods DUSC employs to analyse use of PBS medicines and the PBS data available for these analyses
- outline how the current use of PBS medicines compares with the use as recommended by the PBAC
- support the objectives of the NMP for the quality use of medicines, including supporting consumers' and health practitioners' understanding of the costs, benefits and risks of medicines.[79]

PMR arrangements are:

> 'a systematic approach to monitoring medicines following PBS listing, to inform decision-making relating to ongoing access and subsidy. PMRs provide evidence and options to the PBAC to ensure patient safety, quality use of medicines and the ongoing cost-effective use of PBS-listed medicines'.[80]

While the general principles underpinning the DUSC and PMR arrangements are well accepted, the Review sought to examine (consistent with the Terms of Reference) whether experiences in comparable health systems in respect of health technology reassessment arrangements would be relevant and appropriate to Australia, and how any recommendations arising from the Review could build on the recent procedural changes to the PMR Framework[81] to improve the delivery and operation of such health technology reassessment arrangements.

[77] DHAC (2024) *Post-market Reviews of PBS Subsidised Medicines*, PBS.

[78] DHAC (2022) *National Medicines Policy*.

[79] DHAC (2024) *Drug Utilisation Sub Committee (DUSC)*, PBS.

[80] DHAC (2024) *2024 Post-market Review Framework, PBS*.

[81] DHAC (2024) *2024 Post-market Review Framework, PBS*.

Why this matters

As additional evidence builds over time and new health technologies enter into health care, the original questions asked during an HTA evaluation about clinical effectiveness, cost-effectiveness, clinical need and value for money may have different answers.

A deliberate and structured process that allows these questions to be asked again (via reassessment) is essential to support optimal and judicious use of health technologies over time. This may include changes to subsidy arrangements (e.g. relaxation of access conditions or changes in price).

Structured reassessment processes are therefore an important part of how stakeholders achieve the aims of the NMP in ensuring equitable, timely, safe and affordable access to health technologies for all Australians.

Findings

The Review noted from the paper *Funding and purchasing decisions and Managing Uncertainty*[82] the different approaches to health technology reassessment internationally, and how reassessment arrangements are used to:

- support the management of identified uncertainties from an initial HTA evaluation
- facilitate different outcomes based on the result of the reassessment, including:
  - maintaining current funding arrangements
  - increasing funding for high-value health technologies
  - reducing (or fully withdrawing) funding for low-value or questionable health technologies (i.e. disinvestment[83]).

The Review also noted how the implementation of reassessment and disinvestment arrangements in different health systems reflected:

- the different risks and challenges associated with implementing reassessment and disinvestment arrangements in the localised health settings, including perception, technical and/or procedural and organisational barriers
- the different approaches to achieve passive and active disinvestment, including the use of price adjustment mechanisms, clinical guideline reviews influencing

[82] Centre for Health Economics Research & Evaluation (2023) *Funding and purchasing decisions and Managing Uncertainty*, Health Technology Assessment Policy and Methods Review.

[83] As the generally accepted definition in the HTA context (see International Network of Agencies for Health Technology Assessment and Health Technology Assessment international (HTAi) (2014) 'disinvestment', *HTA Glossary*).

service provider and payer behaviours, and regularly scheduled, systematic programs of health technology reassessment.

In its discussions with clinician and industry stakeholders, the Review also noted that some disinvestment also occurs at an individual health technology and/or localised level. This can be through clinical decisions made between patients and clinicians, such as when observational evidence demonstrates that using a health technology has not provided expected patient health outcomes. However, such disinvestment considerations are rarely reported or collated in a consistent manner to support patient, clinician and healthcare payer stakeholders' decision-making at a broader health system and/or population health level, including via changes to clinical guidelines.

The Review tested the merits of introducing a broader and more comprehensive health technology reassessment program (including an explicit disinvestment framework) that would operate at scheduled intervals to provide funding and disinvestment advice.

Stakeholders generally agreed on the importance of having mechanisms to reassess the use of health technologies after the initial HTA recommendation, consistent with the current operation of the DUSC and PMR arrangements. Suggested improvements to reassessment arrangements included:

- introducing additional flexibility on reassessment timing, depending on the disease area and the rate of health technology development in the clinical space
- extending a health technology's scope to consider repurposing it to address areas of unmet clinical need, and more actively considering re-investment to support ongoing access to important health technologies following the initial funding decision.

However, stakeholders disagreed on the need for a disinvestment framework, or qualified their agreement for a disinvestment framework based on whether:

- they considered the existing DUSC and PMR arrangements were sufficient and appropriate to support these disinvestment considerations by the HTA advisory committee or the healthcare payer
- the reassessment and disinvestment arrangements would apply to all health technologies, or only some health technologies identified via the application of transparent screening criteria
- reassessment and disinvestment arrangements would have the same rigour of analysis and evidence requirements as the initial HTA

- implementation of disinvestment decisions would be sufficiently backed by clear communications and consultations to give stakeholders confidence in the final decisions.[84]

Conclusion

The Review acknowledges the extensive feedback and direct engagement of stakeholders in this matter throughout the Review process.

The Review reiterates its views (reinforced by stakeholder feedback) that some form of health technology reassessment program is necessary and appropriate for the Australian healthcare context to support:

- essential review of new evidence that arises after the initial HTA evaluation (including new clinical data and use statistics) that may inform necessary changes to the original recommendations and decisions to ensure health technology subsidises remain an effective and efficient use of healthcare resources to improve patient health outcomes
- regular examination of a health technology's place in subsidised patient health care, including any significant shifts in uptake, use and/or displacement that may have quality use or clinical practice implications.

Stakeholder support or opposition to health technology reassessment generally reflected different stakeholder perspectives and positions on:

- the purpose of the reassessment (including whether reassessment was for explicit disinvestment and withdrawal of healthcare payer funding)
- whether the core audience of the reassessment outcome is a user, a buyer or a seller of the health technology
- the events or circumstances that have triggered the reassessment process
- the interpretation of what disinvestment means.

In responding to these stakeholder observations, the Review has made recommendations to emphasise the need for the existing mechanisms of ongoing review covering matters of clinical need, use and review of cost-effectiveness, as well as areas where the existing mechanisms can be improved to make a tangible difference to:

- the overall operation of the HTA processes (such as information feedback into activities such as horizon scanning and advice on areas of research need)
- the utility of health technologies for different purposes later in the lifecycle (especially in the context of repurposing and continuing investment to support important availability needs)

[84] See Society of Hospital Pharmacists of Australia, Royal Australian College of General Practitioners, AstraZeneca, and AbbVie submissions to Consultation 2 for examples of diverse perspectives and observations.

- the way that stakeholder engagement and participation is encouraged not just before or during the HTA process, but also during the broader health technology lifecycle, as part of joint responsibility and stewardship for the objectives of the NMP.[85]

Objectives of recommendations

The Review's recommendations are intended to supplement the existing purposes and principles of the reassessment programs and improve decision-making relating to ongoing access to and subsidisation of health technologies.

Recommendations

Recommendation 18. Updated post-Review framework

The Review recommends that the Australian Government:

a. build on existing health technology review and evaluation arrangements (including the Drug Utilisation Sub-Committee (DUSC) and post-market review program) to support regular and periodic examination of the performance, utilisation, displacement and clinical place of a health technology (or health technologies) for a given clinical indication after it has been subsidised by a healthcare payer
b. include activities supporting review throughout a health technology's post-listing utilisation lifecycle, including but not limited to:
    i. advice on commissioning additional health technology–related research in collaboration with existing government programs such as the Medical Research Future Fund (MRFF) or the National Health and Medical Research Council (NHMRC), including (but not limited to) examining the clinical place of, and/or the comparative effectiveness/cost-effectiveness of a health technology
    ii. examination of health technologies for possible repurposing and/or application in other priority sub-populations (including paediatrics) in response to changes in clinical practice, in collaboration with the Therapeutic Goods Administration (TGA) committee(s) supporting the medicines repurposing arrangements
    iii. consideration of appropriate changes to the circumstances required for health technology subsidy
    iv. information for future dialogue with stakeholders on Population, Intervention, Comparator, and Outcome (PICO) horizon scanning and investment or disinvestment considerations (including changes in restrictions) matters, where recommended by a review and supported by the relevant HTA advisory committee

[85] DHAC (2022) *National Medicines Policy*.

v. updates to clinical guidelines and prescribing recommendations for clinicians.

Chapter 5.4: Practical approaches to managing uncertainty and risk, while supporting patient access to health technologies

Introduction and context

The HTA process analyses evidence of the benefits, harms and costs of a health technology to determine its value, including consideration and deliberation on:

- clinical effectiveness – does the health technology work?
- safety – is it safe to use?
- costs – how much will it cost to use?
- economic implications – is it good value for money?
- other information – what are the relevant clinical needs, or social or ethical issues?[86]

Due to the very nature of HTA, the types of questions an HTA tries to answer and the evidence that is available to answer those questions, a level of uncertainty will always exist and hence influence any advice and decision-making about the use of, and funding for, a health technology.

The types of uncertainty that can arise and are most relevant in the HTA context (and consequently need to be considered, addressed and managed by sponsors, HTA advisory committees and healthcare payers) include:

- **clinical uncertainty**, such as how long the clinical benefits of a health technology will last, whether the health technology works as intended in broad clinical practice, and how the health technology compares to existing products or alternatives in terms of clinical outcomes
- **economic uncertainty**, such as the cost-effectiveness of a new health technology (compared with existing health technologies), how the costs compare to the benefits, and whether the cost is justified by the associated improvement in clinical outcomes (quality of life or life expectancy)

[86] DHAC (2022) *HTA for Australian Government subsidy*.

- **Financial uncertainty**, such as the budgetary implications of adopting a health technology and paying for its ongoing use in health care when the true patient population may be over- or under-estimated.[87]

In many cases, the uncertainties identified through HTA processes are – after deliberation and weighing up available evidence – determined to be relatively low or have limited practical consequences. Put differently, the impacts of an adverse outcome arising from the different types of uncertainty identified during the HTA evaluation (such as over-expenditure on a health technology, or a health technology either not providing the expected health outcomes when used in a broader patient population or being cost-effective at a given price) may be relatively low, and therefore does not require specific risk management measures or conditions to be put in place.

However, for an increasing number of health technologies being introduced into health care and put forward for subsidy consideration, the scale and size of uncertainties is much broader and/or can have much more significant adverse consequences if left unmanaged. Examples include:

- health technologies where the quality and/or quantity of evidence available is lower due to smaller patient populations being available for clinical trials. This means the uncertainty of the clinical evidence translating into the broader population is higher, and increases the risk that a health technology will not provide the expected clinical benefits to the patient population
- health technologies that, if subsidised by a healthcare payer, will require very high budget appropriations due to large patient population pools being eligible. This means that the financial uncertainty and risk arising from adopting the health technology is much higher, and could require the diversion of funds from other necessary expenditures to meet patient needs
- health technologies that have high upfront one-off costs and claim to have long-term benefits but have limited follow-up data supporting those claims. This means the clinical and economic uncertainty (i.e. the uncertainty associated with adopting the health technology from health outcomes and 'value for money' perspectives) is much higher, and could result in a higher-risk decision to fund a health technology that in the long run would end up being an inefficient and inappropriate allocation of government funding.

Consistent stakeholder feedback before and during the Review emphasised the importance of having alternative approaches to managing clinical, economic and/or financial impact uncertainties and risks identified during an HTA evaluation. For instance, The New Frontier inquiry discussed at length the use and impact of managed

[87] Centre for Health Economics Research & Evaluation (2023) *Funding and purchasing decisions and Managing Uncertainty*, Health Technology Assessment Policy and Methods Review.

access programs (defined more broadly in international contexts as managed entry agreements (MEAs), discussed further below) as a mechanism to facilitate earlier access to innovative medicines while still ensuring that healthcare payers can demonstrate efficient and effective use of public funds.[88] The New Frontier inquiry also recommended that the Review further examine the issues,[89] and made specific recommendations relating to the use of MEAs.[90]

With this context in mind, the Review examined:

- whether experiences in the way comparable health systems managed HTA-related uncertainty would be relevant and appropriate to Australia
- the operation of the existing MEA framework and stakeholder perspectives raised during The New Frontier inquiry[91] about the operation of MEAs
- the possibility that different approaches to addressing and managing HTA-related uncertainties and risks could help balance the needs and expectations of key stakeholders (patients, clinicians, industry and healthcare payers), while also improving patient access to critical health technologies.

Why this matters

Having the right uncertainty and risk management tools available and using them appropriately can increase the confidence of healthcare payers and industry sponsors translating HTA recommendations into subsidised access to health technologies for patients and clinicians.

Findings

An examination of international practice and dialogue with HTA stakeholders identified two common approaches to addressing and managing uncertainty and risk associated with funding health technologies:

1. at an individual health technology and contract level via MEAs (see Figure 4)
2. at a broader health program level via special funding programs that sit separate to standard healthcare expenditures.

[88] Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

[89] Recommendation 30, Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

[90] Recommendation 10, Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

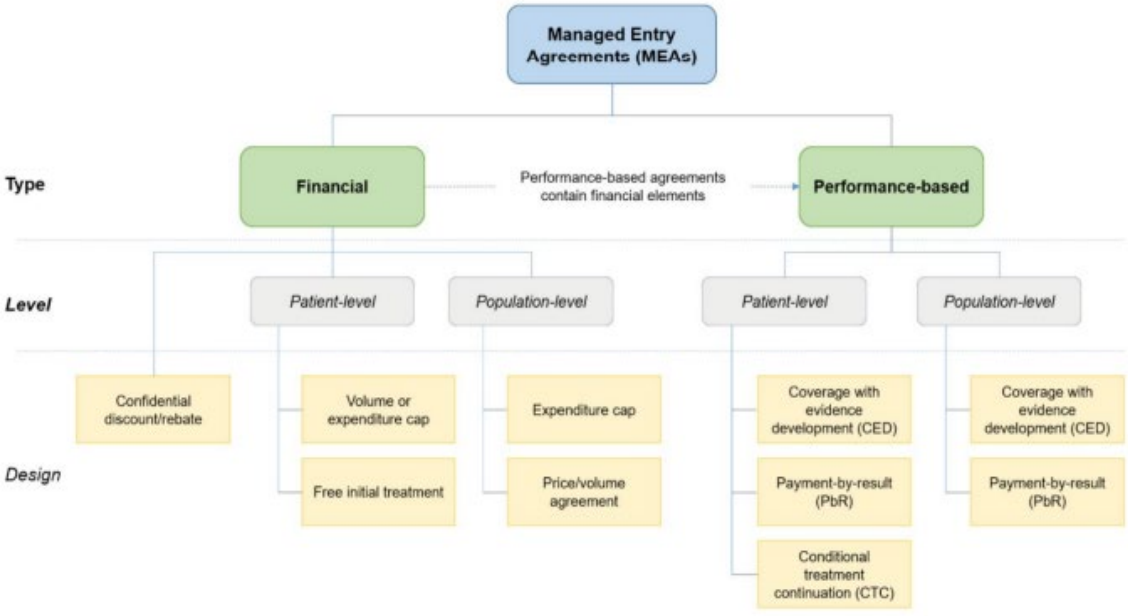
[91] Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

Observations about MEAs

Since a formal framework was introduced for the PBS in 2011, MEAs have been used periodically to address clinical and economic uncertainty.[92] Most have been financial arrangements implemented as part of risk sharing between healthcare payers and product sponsors. However, MEAs have been historically underused. This is partly due to industry stakeholder concerns (though this was not a consistent view) about administrative burdens associated with performance-based arrangements (which often can include collecting patient data to help address uncertainty and risk). Additionally, price points at the commencement of an MEA are low, requiring the sponsor to take a significant financial risk.[93]

In additional consultations, there was consistent support for revising the overall framework for MEAs to improve their uptake and allow for the creative sharing of risk.[94] However, this view was tempered by observations from a small subset of industry and/or health practitioner stakeholders:

- Both types of stakeholder raised the unintended consequences of increased complexity in administering MEAs and their effect on patient access to treatments.
- Industry stakeholders thought the key parties (i.e. healthcare payers and industry sponsors) should share risk.[95]



Source: Wenzl et al. (2019)

[92] DHAC (2011) *Framework for the introduction of a Managed Entry Scheme for submissions to the Pharmaceutical Benefits Advisory Committee*, PBS.

[93] See Janssen's submission to Consultation 1 for a representative view of this issue.

[94] See Roche's submission to Consultation 2 for a representative view of this observation.

[95] See Society of Hospital Pharmacists of Australia and Eli Lilly submissions to Consultation 2 for representative views on this issue.

Figure 4: Managed entry agreements – OECD (Wenzl and Chapman, 2019)

Managed entry agreements

MEAs are strategic arrangements between payers and sponsors to ensure timely patient access to advanced healthcare treatments, particularly when there is uncertainty about their clinical effectiveness or cost-effectiveness. MEAs address uncertainties about the value, uptake and performance of emerging technologies through conditional or managed reimbursement. This means they serve to manage financial risks and other challenges associated with using such treatments, providing a framework for balancing expedited access with efficient resource allocation.

MEAs generally adhere to a three-tier taxonomy (see Figure 4):

- Tier 1 (green) specifies the broad **type** of agreement (i.e. performance-based or financial-based), based on the uncertainty and/or risk to be addressed.
- Tier 2 (grey) categorises the **level** of information that will be analysed and monitored to address the uncertainty and/or risk set out in the agreement (i.e. at an individual patient level, or at an aggregate population level).
- Tier 3 (yellow) defines the **design** of the financial or performance instrument that will be used to manage the uncertainty or risk itself. The instrument can be payments conditional on patient clinical responses, discounts and rebates on the agreed price, or specific volume or expenditure caps (above which additional product is supplied at a different price or at no additional charge).

Observations about special funding programs

A number of Western European countries have established special funding programs (compared to standard funding, and reimbursement and commissioning approaches for health technologies) to provide earlier access to promising health technologies that address a clear unmet need, but have identified uncertainties. Typically, these funding programs have qualifying conditions, or capped expenditures and/or appropriations, and are often restricted to certain health technologies and/or clinical indications (the UK Cancer Drugs Fund and Innovative Medicines Fund are examples of these programs[96]).

[96] A more extensive list of comparable programs is available in Centre for Health Economics Research & Evaluation (2023) *Funding and purchasing decisions and Managing Uncertainty*, Health Technology Assessment Policy and Methods Review.

These special funding programs aim to:

- balance the need to manage the mix of uncertainty and risk identified during the HTA process at a more general health expenditure level (through funding that is distinct from standard healthcare expenditure arrangements)
- provide an avenue for health technologies of high clinical significance to reach patients earlier than standard access approaches (to improve health equity outcomes) while identified uncertainties are resolved as part of further evaluation, stakeholder negotiation or data collection.

Australia **does not** have these types of special funding programs to address these general objectives.

A small set of universal health systems internationally manage risk differently, avoiding the need for (and use of) special funding programs by sequencing their HTA activities and the timing of access to subsidised health technologies differently. These health systems (e.g. in Germany and Japan) support subsidised patient access to a broad range of health technologies at free market prices for a temporary period before an HTA evaluation (and subsequent price negotiation, access adjustments and introduction of uncertainty and risk management instruments).

The Review examined the translatability and applicability of similar arrangements for the Australian healthcare context. It noted that those systems operate under very different healthcare financing and health system delivery structures (being non-profit private health insurance schemes contracting with private entities, operating within a government-defined regulatory framework). They also have distinct social policy, legislative and health system governance structures that prescribe the way price and access to health technologies are negotiated. The differences compared to the Australian health system make the transferability of such concepts much more complex and potentially inappropriate without changing Australia's fundamental approach to health insurance, healthcare financing and health technology access more generally. These issues are beyond the scope of this Review.

Stakeholder consultations on introducing special bridging funding arrangements in Australia to improve access to health technologies revealed strong support across all key stakeholder groups. However, there was some industry and consultant opposition to providing bridging funding via legislated conditional listings arrangements. This was due to concerns that designation of conditional listings would be applied to other health technologies in situations outside the bridging fund context.[97]

[97] See Medicines Australia's submission to Consultation 2 as an example of this view.

Additionally, a small subset of health practitioner and industry stakeholders recommended:

- ensuring careful consideration and design of the specific eligibility criteria (especially regarding time frames, data collection requirements and health technology and/or clinical indication types) to allow sufficient flexibility for important treatments to be eligible for the bridging fund and to limit risks of increased health inequity arising from the selection of eligible health technologies[98]
- having additional design conversations to clarify aspects of operational detail, including how uncertainty and risk would be managed where health technologies exit from a bridging fund, and the timing of funding commencement for health technologies in a bridging fund.[99]

Conclusion

The Review is grateful for the feedback and direct engagement on this particularly tricky issue.

Stakeholder feedback from patients, industry, clinician groups and healthcare payers consistently emphasised the importance of alternative approaches to managing clinical, economic and/or budget impact uncertainties identified during an HTA evaluation. This would enable timely and efficient access to health technologies that address priority health needs.

However, managing uncertainty may require a change in risk posture and engaging with risk in a manner that may be unusual and outside the normally conservative posture the health system and its key stakeholders typically adopt. This is particularly so, given the significant medical, legal and reputational consequences that can result for individuals (illness, injury and death) and the health system overall (inefficient use of limited resources).

The success of any arrangements that try to address uncertainty post-HTA evaluation would require:

- having the right set of contractual or program instruments to manage the different types of uncertainty and address risks, and the willingness to use them creatively given the diverse uncertainties that can arise for any given health technology
- stakeholder willingness to negotiate and participate in managed entry and exit terms and conditions in good faith, and deliver on agreed responsibilities and obligations in a timely and efficient manner.

[98] See Alexion and Assoc. Prof. Steer's submissions to Consultation 2 for representative views of these matters.

[99] See Breast Cancer Network Australia and Novartis Australia submissions to Consultation 2 for representative views of these matters.

There was cautious but generally positive response to options released for consultation that support the management of identified uncertainties. These include:

- creating a separate bridging fund to support earlier access to exceptionally promising, time-critical therapies
- changing the policy and guidance surrounding the use of MEA instruments (including adopting more creative options to address different forms of uncertainty and risk for health technologies, where appropriate).

Internationally, special funding programs are sometimes paired with defined mechanisms for managed entry and/or exit arrangements (linked to finance and performance, as described in the commissioned research papers and summarised earlier in this chapter). Stakeholder visibility and understanding of such mechanisms, generally and at a health technology–specific level, are essential to:

- be transparent about the dynamic tension between:
    - providing temporary patient access to promising treatments to improve health outcomes and health equity across a spread of clinical indications (and not just high-profile diseases such as some cancers)
    - timely re-review of the HTA recommendation to determine if a health technology should move to, or out of, healthcare payer subsidy arrangements so that health resources can be allocated appropriately
    - possible clinical, financial and reputational risks to different stakeholders that may arise if a temporary healthcare payer subsidy is withdrawn due to a final negative HTA recommendation
- support stakeholder confidence in the operation (and resulting outcomes) of the programs, and improve visibility of uncertainty and understanding of why it is important to managing it
- ensure the resources required to support the implementation of MEAs and collect any necessary evidence are proportionate to the uncertainty being managed and addressed, so that participation in these special funding programs is:
    - an **administratively viable** alternative path for sponsors and healthcare payers to resolve identified issues, in contrast to the status quo of addressing issues via multiple HTA resubmissions
    - a **practical** means of improving timely patient access to high added therapeutic value health technologies for critical patient needs
    - **sustainable**, in that final decisions on health technologies are made efficiently to provide stakeholders with the necessary clarity and certainty regarding the use, and funding of, managed health technologies
    - **well supported by stakeholders**, as both active participants and responsible partners in supporting the objectives and outcomes of the MEA (whether they result in positive or negative outcomes in terms of health technology access).

Some stakeholders may be disappointed at the lack of prescriptive detail from the Review about bridging funding program design and operational principles for the revised MEA arrangements. However, the Review:

- did not identify a clear 'best practice' design approach for operating and using a bridging fund for health technologies, as each comparable program experience internationally was established and evolved after extensive consultation and design work to identify the right set of eligibility and operational criteria for the given healthcare context
- considers that how MEAs are contextually applied and used will depend on the uncertainties associated with possible adoption of a health technology; the different approaches that individual healthcare payers or industry sponsors have in engaging with risk; and any other unique commercial or operational implications that are beyond the scope of the Review to examine in detail.

However, in response to stakeholder feedback requesting additional detail, the Review has included additional design principles as signposting elements where it identified commonalities across international programs. Some of these principles may be relevant for the Australian Government as it considers its response to these recommendations and consults further with stakeholders.

In making its recommendations, the Review anticipates that further, ongoing adjustments to the operational principles supporting a revised MEA framework and the operation of a bridging fund will be necessary to account for stakeholder feedback and experience. International experience with the operation of comparable programs demonstrates that such negotiations and adjustments to program parameters are clearly possible. The Review encourages stakeholders to engage constructively on these matters, as the complexities of managing uncertainty are expected to increase in the future.

Objectives of recommendations

The Review's recommendations are intended to support stakeholders engaging with, and managing, uncertainty and risk differently in health technology negotiations and access, so that the process and approach is not a barrier to more timely patient access to important health technologies.

Recommendations

Recommendation 19. Managed entry agreements

The Review recommends that the Australian Government:

a. revise the policy and guidance framework (after consulting with stakeholders) for managed entry agreements (MEAs), to provide more flexibility for sponsors

and the Australian Government to address identified uncertainties while better supporting timely access to health technologies for patients

b. revise the MEA framework to:
    i. provide stakeholders with clarity about processes; for example:
        1. the timing of, and processes related to, the Therapeutic Goods Administration (TGA) and Pharmaceutical Benefits Advisory Committee (PBAC) parallel processing pathway
        2. pricing and negotiation policies that need to be considered as part of settling MEA terms and conditions.
    ii. ensure that the MEA selected for a given health technology considers the complexity of any ongoing monitoring, management and stakeholder engagement by the key parties, and provides the resourcing necessary to support negotiation, administration and communication of the MEA
    iii. ensure transparency and dialogue with stakeholders, including patients and clinicians, on specific access conditions; evidence collection requirements to address clinical, economic and/or financial uncertainties about a health technology identified during the HTA process; stopping rules; and transition processes
    iv. publish key details of the MEA (after necessary redactions) to support transparency of the agreement(s) and stakeholder engagement.
c. if required, seek amendments to legislation and/or regulations to ensure governance and accountability for the MEAs have an appropriate legal basis as recommended by The New Frontier inquiry.

Recommendation 20. Bridging funding program

The Review recommends that the Australian Government:

a. establish (after follow-up stakeholder consultations) a bridging funding program to facilitate earlier, temporary subsidised access to promising, time-critical, therapies of high added therapeutic value that address high unmet clinical need (HUCN) for patients
b. design the program in a way that does not introduce unnecessary complexity into the system, nor create unintended consequences that would prolong assessment, negotiations or implementation of agreed terms and conditions between stakeholders
c. in line with international examples, consider establishing a dedicated but separate budgetary allocation for this program, distinct from baseline Australian Government healthcare funding arrangements (e.g. the Pharmaceutical Benefits Scheme (PBS) and Medicare Benefits Schedule (MBS))
d. include in the program design specific eligibility requirements that health technologies must meet to qualify for temporary bridging funding from this program, in consultation with key stakeholders, including:

i. process-related qualifying requirements – such as earlier submission of health technologies for evaluation via the Therapeutic Goods Administration and HTA parallel submission process – within a defined period after the first major international regulatory approval (e.g. no later than 6 months to 9 months after US Food and Drug Administration or European Medicines Agency marketing authorisation; or within a defined period after identification as part of the HUCN identification process (see Chapter 9.1)
ii. specific conditions a qualifying health technology must meet and that arose from an HTA; for example, where the HTA advisory committee has recommended:
    1. a cost-effective health technology that requires completion of final negotiations and listings processes
    2. a health technology with outstanding issues (resulting in a negative HTA recommendation due to economic evaluation and/or cost-effectiveness) that can likely be resolved quickly, consistent with facilitated and/or early resolution pathway principles
    3. post-HTA recommendation milestones and conditions, such as:
        (a) (if required) provision of additional information that may address identified uncertainties and risks from the HTA evaluation
        (b) agreement on the costs and duration of bridging funding, to ensure the available appropriation allocations are not exceeded
        (c) clear transition pathways and processes (including stakeholder communications) for the health technology to either:
            (i) transition onto standard subsidy arrangements in the case of a positive HTA recommendation and after agreed conditions are fulfilled, or
            (ii) exit from the bridging program, including details on how residual patient needs for the health technology will be met in the absence of further healthcare payer subsidy, if a final HTA evaluation results in a negative recommendation.
        (d) publication (after consultations and necessary redactions) of key terms and conditions for health technologies funded by the bridging program to:
            (i) support visibility of the arrangements and better stakeholder engagement
            (ii) improve patient and clinician participation in addressing specific milestones and conditions.

e. develop appropriate governance arrangements for the bridging funding program, including a scheduled program evaluation to examine whether the program is addressing key objectives.

Chapter 5.5: Antimicrobial health technologies – a multifaceted approach to funding, purchasing and managing uncertainty to improve patient access and availability

Introduction and context

Antimicrobial resistance (AMR) has been recognised by the World Health Organization (WHO) as one of the top 10 global public health threats facing humanity.[100] The rise of AMR has a significant detrimental effect on disease burden and poses a threat to the financial sustainability of health systems and the broader economy globally. While the development of new antimicrobials is critical in addressing AMR, health systems have acknowledged the market failure in new antimicrobials globally due to low return on investment through existing reimbursement methods. Consequently, several large pharmaceutical companies have left antimicrobial R&D.

A WHO review and analysis of antibacterial pipelines in 2021 concluded that recently approved antibacterial agents, and those in the different stages of clinical development, were still insufficient to address the emergence and spread of antimicrobial-resistant infections.[101]

In recent years, Group of Seven countries have engaged in a number of initiatives to create economic conditions to preserve existing antibiotics and their access, strengthen antimicrobial R&D, and bring new drugs to market. UK countries became the first in the world to implement a fully delinked price-volume payment model for antimicrobials following the implementation of a pilot subscription model for two antimicrobials in 2022. Several other countries are at various stages of evaluating and implementing models for encouraging market entry and sustained market availability of high-value antimicrobials.[102]

There has generally been limited guidance from HTA agencies on evaluating antibiotic agents, antimicrobial agents, communicable diseases and infectious diseases.[103] In relation to antimicrobials, the PBAC Guidelines specify that submissions consider the

[100] WHO (2019) *Ten threats to global health in 2019*.

[101] WHO (2021) *Antibacterial agents in clinical and preclinical development: an overview and analysis*.

[102] Global Antimicrobial Resistance (AMR) Research and Development (R&D) Hub and WHO (2023) *Incentivising the development of new antibacterial treatments 2023 [PDF 690KB]*, Progress Report by the Global AMR R&D Hub and WHO.

[103] Adelaide Health Technology Assessment (2023) *Determination of the Population, Intervention Comparator, and Outcome (PICO)*, Health Technology Assessment Policy and Methods Review within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

government-endorsed prudent-use principles proposed in the 1999 report of the Joint Expert Technical Advisory Committee on Antibiotic Resistance and the 'General principles of antimicrobial use' contained in *Therapeutic guidelines: antibiotic*[104] when considering target populations.[105]

A number of considerations for HTAs for antimicrobials have potential implications for HTA processes and policies, including capturing community externalities associated with antimicrobial agents and other infectious diseases. These considerations include accounting for reduced transmission rates, the costs of treating resistant cases and the quality-adjusted life year (QALY) gains from avoiding infection.

In addition to the pilot program for a delinked price-volume payment model for antimicrobials, the UK also trialled a broader value framework – Spectrum, Transmission, Enablement, Diversity and Insurance Value (STEDI) – for evaluating antimicrobials. STEDI is combined with standard HTA dimensions in the UK context (such as clinical effectiveness, costs and safety). This broader value framework was used in the appraisal of the antimicrobials in the delinked price-volume payment model pilot.[106]

As part of its documentation supporting the antimicrobial HTA pilot, the UK's National Institute for Health and Care Excellence (NICE) noted:

- There can be difficulties in defining the relevant population and subgroups for antimicrobials, as market authorisation may focus on pathogens rather than clinical indications.
- In determining the population for antimicrobial use, the setting (community, hospital or restricted to intensive care use) should be considered, as the rate of infections and transmission dynamics will differ based on this.
- The benefits of antimicrobials can extend beyond the patients treated to the wider population, so the outcomes considered for each HTA evaluation need 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

[104] Therapeutic Guidelines (2019; amended 2023) Therapeutic Guidelines: Antibiotic Version 16, Melbourne: Therapeutic Guidelines Limited

[105] See 5.3 in DHAC (2016) *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee.*

[106] Schurer M, Patel R, van Keep M, Horgan J, Matthijsse S and Madin-Warburton (2023) 'Recent advances in addressing the market failure of new antimicrobials: Learnings from NICE's subscription-style payment model', *Frontiers in Medical Technology*, 5:1010247, doi: 10.3389/fmedt.2023.1010247.

the pathogen, or based on clinical suspicion of the pathogen and its mechanism of resistance).

Taken together, these observations from the UK pilot are consistent with the general view that providing access to new antimicrobial health technologies in health care is likely to require a multifaceted and distinct approach to HTA evaluation, managing uncertainty, negotiations (on price and specific contractual terms and conditions) and subsequent reassessment activities (in response to resistance and transmission dynamics at different points in time) compared to other health technologies.

The Australian Government has released *Australia's National Antimicrobial Resistance Strategy – 2020 and Beyond* (2020 Strategy), which was endorsed by the Council of Australian Governments on 13 March 2020. It sets a 20-year vision to protect the health of humans, animals and the environment through minimising the development and spread of AMR while continuing to have effective antimicrobials available.[107]

The 2020 Strategy recognises that stewardship practices have reduced the demand for antibiotics worldwide; however, this has also contributed to the lack of supply of new antibiotics as there is a lack of incentive (financial) for pharmaceutical companies to develop them relative to other medicines. The 2020 Strategy recommended funding and subsidy approaches that incentivise the development of new antibiotics while maintaining responsible stewardship (i.e. restricting their use to appropriate use). There are opportunities across the HTA continuum to explore options to incentivise development, including in areas such as how government assesses, funds and subsidises new antimicrobials.

The Government's response[108] to The New Frontier inquiry noted that the Department has commenced work towards identifying and scoping potential funding mechanisms and economic models to incentivise market availability of antimicrobial products in Australia.

In 2015, the OECD released a paper on the rise in AMR in G7 nations and the associated population health, social and economic consequences.[109] The paper expressed concerns about AMR as a global health challenge requiring a global, coordinated effort to implement comprehensive action plans. The OECD recommended taking an international approach to foster innovation and lower barriers that hinder R&D in the antimicrobial sector and to increase the productivity of research globally. The paper recommended that this approach combine upstream and downstream economic

[107] Australian Government (2020) *Australia's National Antimicrobial Resistance Strategy – 2020 and Beyond*.

[108] Australian Government (2023) *Government response – The New Frontier: Inquiry into approval processes for new drugs and novel medical technologies in Australia*.

[109] Cecchini M, Langer J and Slawomirski L (2015) *Antimicrobial Resistance in G7 Countries and Beyond*, OECD.

incentives to delink development incentives from sales, and encourage the participation of small to medium-sized enterprises in R&D efforts.

The availability of antimicrobial health technologies in the Australian market was relevant to the Review. It found opportunities across HTA processes and policies that could be changed to support greater investment and remove barriers in the evaluation of such health technologies for market authorisation and public subsidy.

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. These include accounting for reduced transmission rates, the costs of treating resistant cases, the QALY gains from avoiding infection, and performing sensitivity analysis on different levels of resistance.

Findings

Stakeholders considered that providing HTA fee exemptions for health technologies for AMR could help address the market failure in antimicrobials if implemented as part of a package of reforms to improve the market viability of antimicrobials. Stakeholders supported establishing alternative funding arrangements to incentivise the development of new antimicrobials and bringing these to market in Australia. Industry stakeholders said alternative funding arrangements should be implemented with urgency and that further deliberation could delay access to novel antimicrobials. It was also unnecessary given the available information on this matter.

There are issues in Australia and globally regarding the limited commercial incentives to develop certain types of products, such as new antimicrobials. This is contributing to a decreasing toolkit globally to address AMR. It is an exceptionally complex multifaceted issue, clinically and economically, and requires a multifaceted approach to incentivise the development and marketing of AMR technologies in Australia and globally.

Stakeholder submissions raised concerns about the significant lack of incentives for developing products that address AMR.[110] Action is required to reduce disincentives for organisations seeking funding for antimicrobials. The Government should also investigate what changes could be made across the HTA continuum, including to funding arrangements to incentivise the development of new antimicrobials.

Alternative approaches to HTAs for antimicrobial health technologies are being developed and tested in overseas jurisdictions. This included modifying definitions in the Population, Intervention, Comparator, and Outcome (PICO) criteria, approaches to evaluating evidence, and dimensions of value that are considered when evaluating these health technologies. Of particular note, researchers, health policy agencies and

[110] See Society of Hospital Pharmacists of Australia's submission to Consultation 2 for a representative view on this matter.

HTA agencies are trending towards acknowledging the broader implications for indirectly protecting population health through the proper use of antimicrobial health technologies. In addition, it is important to consider the positive longer-term health system effects associated with the availability of a broader suite of narrow-spectrum antimicrobial health technologies, rather than over-reliance on broad-spectrum products that may accelerate resistance trends. This in turn will help in managing future AMR risk.

Conclusion

The Department has begun work on identifying and scoping potential funding mechanisms and economic models to incentivise market availability of antimicrobial products in Australia. It is important that future work on changing the HTA continuum should be informed by this existing work.

Significant additional work is required to design and implement a pilot and policy changes to make an impact on AMR. The Department would require additional resourcing to implement this work.

Alternative payment and reimbursement reforms are being tested internationally. Some stakeholders expressed keen interest in delinking price and volume for antimicrobial products, given the need to balance the public health policy approach to product stewardship to address AMR in the long term.

Given the different AMR patterns in different countries, options for alternative payment and reimbursement reforms for antimicrobial products will need to be relevant to the Australian health system context. This includes current Commonwealth and jurisdictional funding structures for health technologies. These should also be broadly consistent with global strategies providing incentives to bring antimicrobial products to market.

Objectives of recommendations

The Review recommends implementing practical changes and investments that improve incentives to develop new antimicrobials and contribute to the global fight against AMR. It recognises that addressing the need for health technologies to fight AMR is an exceptionally complex multifaceted issue that requires a multifaceted approach.

Recommendations

Recommendation 21. Approaches to incentivise the development of health technologies that address antimicrobial resistance (AMR)

The Review recommends that the Australian Government:

a. exempt antimicrobial health technologies that target organisms on the World Health Organisation (WHO) bacterial/fungal priority pathogen lists, and that are identified to be important for addressing public health risks in Australia, from HTA fee requirements
b. examine, consult on and develop a framework to inform changes to HTA policy and methods for antimicrobials, given the public health significance and implications of AMR. The framework should be informed by existing work undertaken on identifying and scoping potential funding mechanisms and economic models to incentivise market availability of antimicrobial products in Australia
c. design a flexible reimbursement policy for antimicrobial products. The policy should examine and test multiple payment and incentive models, including but not limited to full and partial price and volume delinking, advanced market commitments and guarantee-of-supply provisions
d. in the short term, develop, implement and assess the effectiveness of a pilot subscription fund for novel antimicrobials. The model should be guided by international examples but tailored for the Australian setting. The pilot should also be guided by recommendations from The New Frontier inquiry.

Chapter 6: Transparency and stakeholder involvement

Introduction

Effective stakeholder engagement is a key contributor to organisational resilience and flexibility, learning and innovation, the identification of new opportunities, and ultimately to sustainable performance.[111] Stakeholder engagement depends on two-way communication, which requires transparent and clear communication of processes, policies and decisions at its core.

Transparency and effective stakeholder engagement have many benefits for government, individuals and society more broadly. For government, transparent and clear public communication improves accountability to all stakeholders, builds trust and confidence in government decisions, and improves service delivery and efficiency.[112] For individuals and organisations, the ability to access information on government processes and policies enables them to more effectively and meaningfully participate in decisions that impact them. Additionally, transparent and coherent communication of information enables individuals and companies to make informed decisions and plan for their future appropriately.

Stakeholder engagement benefits HTA by contributing additional information that may help to address gaps in evidence, validate claims, clarify consequences and aid the interpretation of evidence, especially for the local setting.

The Department characterises effective stakeholder engagement as two-way open communication that involves listening to stakeholders, keeping them informed and being clear about how their contributions are being used.[113] Similarly, transparency is more than sharing information or data. It includes keeping people informed and supporting them to understand matters that are important to them. This means information needs to be fit-for-purpose and easily accessed and understood by all stakeholders.

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[111] AccountAbility (2005) AA1000SE Stakeholder Engagement Practitioner's Perspectives from DHAC (2017) *Stakeholder Engagement framework*.

[112] Office of the Australian Information Commissioner (2021) *Statement of principles to support proactive disclosure of government-held information*, Office of the Australian Information Commissioner website.

[113] DHAC (2017) *Stakeholder Engagement framework*.

Partnership, collaboration, cooperation and transparency are key themes underpinning the NMP. They are integral to achieving the aim of the policy and the intended outcomes of each of its central pillars.[114] The success of the NMP relies on shared decision-making, strategic partnerships and the involvement of people with lived experience in the co-design, development, implementation and evaluation of related policies, strategies, programs and initiatives. The Review participants identified the importance of achieving the intended outcomes of the NMP and complementing the co-design of an Enhanced Consumer Engagement Process.[115] The latter project focuses on access to earlier information about new health technologies and improving the understanding of consumer issues before a medicine is listed.

The principles of communication, transparency and stakeholder engagement are fundamental features of existing Australian HTA processes. However, multiple issues were identified that require solutions to improve communication and transparency, and optimise how patients, consumers, health professionals and other stakeholders engage across all HTA processes. The Review therefore recommends a range of initiatives across the HTA continuum to better engage with those who are affected by HTA decisions and to improve transparency.

This chapter covers the Review's consideration and recommendations:

- Transparency and communication of HTA pathways, processes and decisions (Chapter 6.1)
- Consumer, clinical and other stakeholder involvement and consideration in HTAs (Chapter 6.2)
- Development of an explicit qualitative value framework (Chapter 6.3).

Note: First Nations people's involvement and consideration in HTAs requires holistic solutions integrated across the HTA continuum (see Chapter 3.1).

Chapter 6.1: Transparency and communication of HTA pathways, processes and decisions

Introduction and context

The Department publishes information about HTA pathways, processes and decisions on various websites, including those for the PBS[116] (and Medicines Status Website[117]),

[114] DHAC (2022) *National Medicines Policy*.

[115] DHAC (2024) *Co-design of an Enhanced Consumer Engagement Process for health technology assessment*.

[116] DHAC (2024) *Pharmaceutical Benefits Scheme (PBS)*, PBS.

[117] DHAC (2024) *Medicine Status Website*, PBS.

the MBS,[118] the PBAC,[119] the MSAC[120] and the Consultation Hub.[121] Information is targeted for patients, clinicians and sponsors.

The PBAC[122] and the MSAC[123] Guidelines provide information on how to prepare a submission for an HTA, including proposed use of the medicine, technology or service, clinical evidence, economic evaluation, extent of use and financial estimates. The Guidelines include additional relevant information that may influence decision-making, such as use of expert opinion (from clinicians or consumers) or issues of equity, ethics, access or public health. A plain language summary of the MSAC Guidelines is available for stakeholders.[124] Process information for listing medicines on the PBS is presented in a procedure guidance document[125].

Agendas for PBAC and MSAC meetings are published in advance to facilitate consultation input from stakeholders. Any individual or organisation can provide input via a consultation survey or by uploading a separate file. For submissions considered by the PBAC only, the submission title is published. For the MSAC, the application form and PICO scoping are published, providing more information to inform the consultation.

HTA advisory committee advice, recommendations and decision rationales are published in public summary documents (PSDs).[126,127] The MSAC PSD includes a consumer summary. PBAC Outcomes, with a brief summary of the decision, are published ahead of the PSD.

Participants in the Review described the information published about HTA pathways, processes and decisions as extensive, but 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 navigate, presented in technical language, or deals with concepts that are not explained well. Further, stakeholders are not satisfied with existing plain language explanations of HTA pathways and guidelines. The Review also found that there are many misconceptions about the HTA system and its processes, further demonstrating a need for plain language communications and opportunities to enhance clarity and understanding.

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[118] DHAC (2024) *MBS Online*, Medicare Benefits Schedule (MBS).

[119] DHAC (2024) *Pharmaceutical Benefits Advisory Committee (PBAC)*, PBS.

[120] DHAC (n.d.) *Medical Services Advisory Committee (MSAC)*, MSAC.

[121] DHAC (2024) *Office of Health Technology Assessment Consultation Hub*.

[122] DHAC (2016) *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*, PBS.

[123] DHAC (2021) *Guidelines for preparing assessments for the Medical Services Advisory Committee*, MSAC.

[124] DHAC (2021) *MSAC Guidelines Summary for Stakeholders*, MSAC.

[125] DHAC (2020) *Procedure guidance for listing medicines on the Pharmaceutical Benefits Scheme*, PBS.

[126] DHAC (2024) *Public Summary Documents*, PBAC.

[127] DHAC (2016) *Post MSAC Process*, MSAC website.

What we heard:

> An overarching theme affecting many aspects of the performance of the HTA system was transparency with respect to how reimbursement and/or pricing decisions are made, the factors (evidence) that are considered in making those decisions, and in some cases understanding the overall steps in the HTA. While many stakeholders considered that the process for an HTA in Australia was generally well described, it was not always clearly understood.
>
> For some, the language used to describe HTA processes and requirements was overly technical, obfuscating their nature. For others, there was a lack of information on specific aspects of processes (such as how evidence was being combined or weighted in reaching decisions about reimbursement and/or funding) or no public visibility, impeding engagement with the system and potentially resulting in poorer access.
>
> **Summary sentiment from Consultation 1 report**

The Review heard from organisations representing patients and consumers that they found it difficult to prepare submissions due to insufficient information regarding the sponsor submission in the agenda documents (particularly for PBAC submissions).

Findings

More extensive or clearer information is needed in specific areas to optimise stakeholder engagement. This includes plain language summaries of PBAC submissions and outcomes and explicit advice about how evidence that does not form part of the clinical or economic evaluation is considered in decision-making. Presentation of information about submissions under consideration or completed should be improved to aid understanding of how the system is performing.

What we heard:

> 'The Options paper produced provides for an extensive range of adjustments and reforms, which together provide a positive roadmap for change. The paper includes a strong focus on reforms which can increase consumer engagement and given our role as a patient organisation, we strongly welcome this focus.'
>
> **Consultation 2 submission: Australian Patients Association**

Stakeholders were emphatic about the importance of engaging broadly, co-designing plain language summaries, improving the websites and developing a dashboard to meet the needs of consumers and all stakeholder organisations.

The paper *International Health Technology Market Approval, Funding and Assessment Pathways*[128] found that decisions were completely transparent for sponsors and partially transparent for patients in Australia. PSDs commonly contain redactions of clinical and economic evidence. However, transparency was defined by the availability of information, not by the ability of the community to make sense of that information.

The Review also found that inherent and structural aspects of the HTA process work against transparency and may limit maximum engagement. These factors include the complex nature and volume of information considered by HTA advisory committees, the large and increasing number of applications received, the need to protect commercially sensitive or an individual's private information from public release by law, and engagement usually not occurring until after an HTA submission is made.

Plain language summaries

Stakeholders strongly supported having plain language summaries to facilitate greater stakeholder and consumer understanding, engagement and input. Consumer, clinical and industry organisations agreed they would have a positive or very positive impact.[129] Consumer organisations noted that introducing plain language summaries, or improving existing summaries, in the near term would have a substantial positive impact.[130]

Consumer and industry participants recommended that plain language summaries of medicine submissions could be provided alongside upcoming PBAC agendas. One industry stakeholder noted that criteria were needed to define which submissions would benefit from having a formal summary.[131] Industry stakeholders also considered that there was a strong need to keep pricing information confidential, in accordance with the law, where applicable.

Plain language summaries of HTA advisory committee decisions (including a clear description of how stakeholder perspectives were taken into account in relation to other inputs and evidence) were also considered a very important element of effective engagement and had widespread stakeholder support.

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[128] Adelaide Health Technology Assessment (2023) *International Health Technology Market Approval, Funding and Assessment Pathways*, Health Technology Assessment Policy and Methods Review within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

[129] Bastion Insights (2024) Consultation 2 Stakeholder Engagement Report, DHAC.

[130] Consensus Letter from 51 consumer organisations to Consultation 2.

[131] Bristol Myers Squibb Australia's submission to Consultation 2.

Consumer organisations recognised that when conveying HTA decisions, much of the information is very technical, which can be difficult for patients to grasp. Publishing plain language summaries enables transparency and equity of consumer access in the context of diversity in health literacy. Plain language summaries may still fail to communicate the complexity of decision-making processes as they rely on a certain level of health literacy. A concern was raised about the risk of increasing inequities unless there is an effort to improve health literacy across affected consumer groups. Further, an appropriately skilled workforce that includes scientific and medical communications experts is needed to support clear communication of HTA advisory committee decisions and rationale.

HTA website

Stakeholders welcomed improvements and upgrades to the HTA website and stated that a dashboard would be a valuable new inclusion. Co-design of new inclusions was considered crucial. Reported benefits included improved information dissemination; enhanced understanding; more transparency around processes; and more options and opportunities for stakeholders to provide input. Other benefits include increasing accountability on the progress of individual submissions; encouraging proactive preparation of the health system for new or amended medicines on the PBS; and improving the searchability of submissions.

However, these reforms could also have unintended consequences. Various stakeholders expressed concern about the resources need to create a new dashboard, update the websites and produce plain language summaries. A commensurate increase in funding and/or resourcing would be required to avoid the risk of diverting resources from the HTA process, potentially impacting the timely assessment of HTA submissions.

Conclusion

Information needs to be suitable for a range of audiences, and it needs to be consolidated and linked. Aspects of the HTA system are complex, and communications methods, formats and content benefit from co-design and user testing to ensure they meet their intended purpose.

Objectives of recommendations

The Review's recommendations will improve communication and transparency by providing relevant, accessible information about HTA policy, processes, submissions and outcomes. This will enable the diverse range of stakeholders with differing perspectives and knowledge of the HTA system to fully participate and understand decisions that impact them. This is intended not only to improve HTA outcomes but also enable consumers to have more realistic expectation of the proposed treatment benefits and populations of a proposed therapy to make informed decisions. The

recommendations will also improve transparency on the progress of individual health technologies through the HTA system and overall system performance.

Recommendations

Recommendation 22. Publishing plain language summaries

The Review recommends that the Australian Government:

a. make plain language summaries of Pharmaceutical Benefits Advisory Committee (PBAC) submissions available at the same time as the PBAC agenda. The summaries would have to be developed in collaboration between sponsors and the Department of Health and Aged Care. Information included should allow consumers (including patient communities and clinicians) to be better equipped to provide input to the HTA process. Additionally, they should provide information for patient communities to understand the expected benefit of the therapy and the proposed population, without ambiguity. Over time, with the earlier engagement of consumers, these summaries may evolve with the consumer along the health technology pathway
b. develop clear, unambiguous and transparent descriptions of committee deliberations that can be understood by patient communities. This includes clear reasoning for recommendations and/or decisions made, and factors affecting decisions (see 'Recommendation 26: Developing an explicit qualitative values framework') including enabling consumers to see how their input was considered and factored into the decision. These should be published where possible or otherwise disseminated broadly to stakeholder groups.

Recommendation 23. Improving the HTA webpage including developing a dashboard

The Review recommends that the Australian Government:

a. enhance access to information about processes, policies and decisions on the HTA website by:
    i. improving navigation
    ii. using accessible language
    iii. tailoring information to specific stakeholder groups where appropriate; for example, clinicians, consumers, health organisations, individual patients and carers, industry and sponsors
    iv. presenting information in a variety of formats, using aids such as case studies and infographics to explain complex topics to stakeholders with differing levels of HTA experience.

b. develop a user-friendly, data-driven, online information platform that makes it easier to find out about HTA processes, outcomes and performance, and includes:
    i. a visual data dashboard for statistics and metrics
    ii. information on individual therapies at each decision point or key milestone
    iii. clear reasons for delays or decisions (including those made by the Government and sponsors) including:
        1. standardised reasons for Pharmaceutical Benefits Advisory Committee (PBAC) outcomes for non-recommended therapies (can be multiple reasons)
        2. standardised reasons for delays in listing therapies after PBAC recommendations
        3. planned implementation timelines for highly specialised therapies (see 'Recommendation 13. Improved processes, accountability and timeliness for highly specialised therapies and other therapies co-funded between the Australian and state and territory governments'), including reasons for any delays on expected time frames.
    iv. aggregated information about timelines and decisions, such as for all applications, all medicines claiming additional clinical benefit, therapies for particular indications, and classes of therapies
    v. capacity to link information for a therapy across the HTA pathway, including from the Australian Register of Therapeutic Goods (ARTG) application to Pharmaceutical Benefits Scheme (PBS) listing, with consistent standardised recording of indications, populations and drug name
    vi. information about when new medicines or expanded indications are first launched globally relative to when they apply for ARTG registration and PBS listing.
c. provide information about the outcome of any proactive submission sought by the Government (see 'Recommendation 46. Proactive pre-HTA processes supporting introduction of identified health technologies for high unmet clinical need').

Chapter 6.2: Consumer, clinical and other stakeholder involvement and consideration in HTAs

Introduction and context

Stakeholder engagement in HTAs can involve engaging individuals, representatives or groups of patients, carers, clinicians, sponsors and industry, peak organisations,

academics and subject matter experts, government bodies, and other members of the public.

For the PBAC and the MSAC processes, consumers, health practitioners and any other interested groups or organisations can 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).

As noted in the *Conversations for Change report*,[132] the visibility of patient involvement and consumer representation in HTAs has increased in recent years. In 2017, the HTA Consumer Consultative Committee was established, followed by the Department's Consumer Evidence and Engagement Unit in 2019. These initiatives have improved the ways consumers and patients engage with HTA processes and are seen as elements of the system that are working effectively.

The Review 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 submissions.

Stakeholders also felt that engagement with patients and consumers was not occurring early enough, both during 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 earlier to enable them to help identify priorities and enable greater understanding of lived experience.

Findings

Having an engagement framework that spans the HTA continuum would help with realising the full potential value of stakeholder involvement.

Review participants conveyed that sustainable and systemic support is essential to establish and embed engagement. A lack of feedback on how input is used prevents consumers and their organisations knowing whether the considerable time they invested in providing input was worthwhile as they do not know how it was considered or how it could have been improved. Stakeholder engagement mechanisms must be

[132] DHAC (2023) *Conversations for Change report: improving consumer engagement in Health Technology Assessment*.

co-designed to ensure that individuals and organisations, irrespective of size, have the capacity and opportunity to participate in HTA processes. Several stakeholders raised the importance of having adequate funding and resources for consumer organisations to engage equitably in HTA processes.

Having a stakeholder engagement framework would also enable health professionals to clearly articulate and augment HTA processes.

What we heard:

> 'We support any measures that increase community member and healthcare professional engagement, ensuring that this occurs as early as possible and frequently throughout the process, and all outputs or decisions made because of that input are transparently shared with the community.'
>
> **Consultation 2 submission: Cancer Council, Cancer Nurses Society of Australia, the Clinical Oncology Society of Australia, Private Cancer Physicians of Australia and Medical Oncology Group of Australia**

Many consumer organisations – including the Consumers Health Forum of Australia, the Australian Patients Association, and 51 consumer-led organisations (which provided an unprecedented collaborative response) – considered that the value of the consumer voice in HTA policy and methods should be enshrined in legislation. This would ensure that consumers and their knowledge (needs, preferences, experiences, and perspectives) were safeguarded as a cornerstone of the Australian HTA system. This would also maintain the existing consideration of consumer perspectives and improve it over the long term. Currently, one or more PBAC members can represent consumers on the PBAC (section 100B of the NHA[133]), safeguarding advocacy for patient needs, preferences and perspectives. But more work needs to be done to:

- establish what additional benefits legislation would offer beyond procedures, policies and guidelines – particularly the ability to modify them if they are not achieving their intended purpose
- how enshrining a voice should be given effect – that is, how should legislation be amended, what change should be made to the existing powers and functions under the NHA.

A stakeholder engagement framework should:

- be consistent with the principles of the Enhanced Consumer Engagement Process to:

[133] *National Health Act 1953*.

    - prioritise consumer evidence and experience, and making it integral to HTA processes
    - ensure that recommendations that improve consumer engagement do not delay access to health technologies
    - prioritise enhancements for consumer engagement that achieves maximum impact through implementation.
- include approaches that improve clinician engagement
- make visible equity and inclusion of First Nations communities
- support and emphasise equity and inclusion of socially, culturally and linguistically diverse communities and under-represented groups
- describe how and why engagement is used to support HTAs and improve patient outcomes across the system, and all relevant processes, including:
    - horizon scanning, PICOs, pre-submissions, evaluations, appraisals, PMRs and disinvestment in health technologies
    - the wider health technology pathway of clinical research, other evidence development and regulation
    - co-design of new processes and tools arising from the Review
- develop opportunities for dialogue including real-time interaction at committee meetings
- set out the requirements to support this engagement including through:
    - proactive identification of consumers
    - audience- and touchpoint-specific plain language summaries co-developed to evolve with the process
    - consumer subgroup requirements, especially considering those that do not engage with the online submission portal, and which may require co-designed solutions
    - training and capacity building for external stakeholders to improve understanding of HTA processes
    - training and capacity building for HTA staff, committee members and evaluators to improve understanding of consumer evidence and engagement
    - clear and transparent guidance about how input should be prepared, how it will be used by committees, and approaches for managing confidentiality and conflicts of interest
    - clear reporting to groups about how their input has been used (such as through a values framework and briefings)
    - plans for process review and continuous improvement
    - how consumer evidence and engagement elsewhere in the health technology process will be transparent to engaged consumers and how their engagement will be documented for later processes
    - adequate resourcing for engagement and support.

Conclusion

Increasing the involvement of consumers and clinicians earlier and more consistently and formally throughout the HTA pathway would improve the performance and person-centredness of the HTA system. When stakeholders (e.g. consumers and clinicians) are only engaged after an HTA submission has been lodged, it can leave inadequate time to resolve implementation challenges, leading to delays in accessing health technologies. It can also result in the submission missing outcomes that are important 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 knew what information would be most useful to the HTA advisory committee and how to best present that information.

Formalised feedback in an easily understood format about how stakeholders' submissions contributed to HTA decisions is also lacking. While the Consumer Consultative Committee performs this process, it has limited resources, and it is not undertaken as a standard course of action.

Consultation 2 tested options with stakeholders aimed at improving communication of HTA pathways, processes, decisions and stakeholder engagement. Increasing transparency and stakeholder involvement is linked to improved efficiency and service delivery. Many options that improve transparency or inclusiveness are also likely to have other desirable outcomes such as timely access and efficiency.

The development of options was informed by, and integrated, the rich consultation already undertaken for the Conversations for Change consultation and report, key outcomes of The New Frontier inquiry,[134] and the Review literature analysis and consultations. The Review was also cognisant of the proposed recommendations for the co-design of the Enhanced Consumer Engagement Process.

As a result of extensive consultation, most options that proposed to improve communication, transparency and stakeholder engagement reflected stakeholder views. They were strongly supported by stakeholders and have been adopted in the Review's recommendations. Stakeholders considered that a number of the recommendations could be implemented relatively quickly, and have an immediate impact.

Objectives of recommendations

The Review's recommendations to improve communication, transparency and stakeholder engagement are multifaceted and person-centred, and include short-,

[134] Australian Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

medium- and long-term initiatives. They aim to optimise timely and meaningful engagement by individuals and organisations across the HTA continuum through the use of plain language communications, an end-to-end engagement framework and improved data to monitor progress and performance.

Recommendations

Recommendation 24. Developing an engagement framework

The Review recommends that the Australian Government develop a stakeholder engagement framework that is guided by the recommendations of the Co-Design of an Enhanced Consumer Engagement Process, this Review, The New Frontier inquiry and *Conversations for Change report*. This framework should describe how and why engagement with stakeholders is used across all HTA processes, from horizon scanning to post-market review. The framework should focus on consumers, including co-design of engagement processes for under-represented communities. Additionally, the framework should acknowledge that the policies, methods and decisions for the HTA pathway have impacts throughout the whole health technology lifecycle and can be used to improve stakeholder engagement outside the direct HTA processes.

Recommendation 25. Improving involvement of consumers in HTAs

The Review recommends that the Australian Government, in addition to other recommendations to improve engagement, inclusion and use of consumer evidence, support consumers to engage with HTA processes through:

a. actively engaging consumers across the HTA system and all relevant processes including horizon scanning; the Population, Intervention, Comparator, and Outcome (PICO) scoping; pre-submissions; evaluations; appraisals; post-market reviews, and disinvestment decisions
b. updating the Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines to specifically request information about how consumers were engaged in the pre-HTA processes including clinical trial design
c. developing education and training to improve consumers' ability to understand and engage with the HTA processes, including how input should be prepared, and how it will be used by committees.

Chapter 6.3: Development of an explicit qualitative value framework

Introduction and context

Review participants raised a common concern that PSDs do not sufficiently communicate the influence certain types of evidence, such as patient and consumer input, has on decisions to recommend or not recommend funding of a health

technology. There was agreement that better communication of the factors beyond clinical effectiveness, safety and cost-effectiveness that are considered by HTA advisory committees, and their influence on decisions, would enhance transparency and understanding.

The PBAC[135] and the MSAC[136] Guidelines state that decision-making is informed by less readily quantifiable factors beyond clinical effectiveness, safety and cost-effectiveness. These include equity, clinical need, severity, the value of knowing prognosis or diagnosis, public health issues and other relevant considerations. In this sense, the PBAC can be considered as making value-based assessments that incorporate broader value domains qualitatively rather than quantitatively. However, there is no guidance on how to present that information, nor how HTA advisory committees consistently consider and apply that information in their decisions.

Broader value elements are considered by most HTA agencies around the world,[137] but their role and effect on decision-making lack transparency. Several studies identified in *Clinical Evaluation Methods in HTA*[138] 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 *Clinical Evaluation Methods in HTA*:

- qualitative MCDA (qualitative value framework)
- quantitative MCDA (each value element for decision-making is scored and then synthesised using predetermined weights, and an overall score is generated)
- MCDA with decision rules (i.e. value-based decision rules).

The use of MCDA in HTAs is not widespread. *Clinical Evaluation Methods in HTA* found that only one jurisdiction (NICE) used a formal weighting of benefits approach (QALY weightings that differ from the reference case) in decision-making. Canada's Ontario jurisdiction did not endorse the MCDA approach, finding that structured decision-making introduced undesirable rigidity into the process. Many countries used a more informal approach, taking other information, such as consumer experiences and equity

[135] DHAC (2016) *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*, PBS.

[136] DHAC (2021) *Guidelines for preparing assessments for the Medical Services Advisory Committee*, MSAC.

[137] Adelaide Health Technology Assessment (2023) *Clinical Evaluation Methods in HTA*, Health Technology Assessment Policy and Methods Review within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

[138] Adelaide Health Technology Assessment (2023) *Clinical Evaluation Methods in HTA*, Health Technology Assessment Policy and Methods Review within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

issues, into consideration during decision-making. Equity is usually considered in a deliberative manner by appraisal committees.

Findings

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, the value of knowing prognosis or diagnosis, public health issues and other relevant considerations – are being considered by HTA advisory committees and what impact they have on decision-making.

Other jurisdictions have developed explicit value frameworks for this purpose. In this context, 'value' means consider important. The term 'explicit' refers to the value elements the committee considers, how they consider them, and their known impact on decision-making.

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

Patient, consumer, industry and clinical organisations felt that developing an explicit qualitative value framework would have a positive or very positive impact for them or their organisation and would provide transparency and additional context around decision-making.

Review participants thought the framework should be developed in consultation with stakeholders. Some industry stakeholders said the framework should be developed separately from HTA advisory committees. This view was not shared by other stakeholder groups.

The option proposed in the Review focused on making more explicit how the currently considered less quantifiable elements – such as patient and consumer input, equity, clinical need, severity, the value of knowing prognosis or diagnosis, and public health issues – influence HTA advisory committee decision-making.

There were mixed views on the range of elements that should be represented in the qualitative framework. Some stakeholders, mainly industry participants, explored the inclusion of a much broader range of elements: societal benefits such as productivity benefits, reduced carer burden, treatment choice and real option value (e.g. life-extending treatments that may increase treatment options in the future). The PBAC Guidelines include a dedicated appendix describing how non-health benefits, such as productivity, can be included as a supplementary quantitative analysis in addition to the base case.[139]

[139] See Appendix 6 in DHAC (2016) *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*.

All stakeholders consistently highlighted consideration of equity and priority populations, such as First Nations people, as an essential element for inclusion. It was suggested that a checklist should be developed to consistently assess health inequality impacts in Australia.

Conclusion

The absence of guidance and other communications demonstrating how wider elements (beyond clinical effectiveness, cost-effectiveness and financial impact) are considered by HTA advisory committees has created uncertainty about the influence they have on decision-making.

Objectives of recommendations

The Review's recommendations aim to ensure that the wider elements HTA advisory committees take into account in deciding whether a health technology should be funded are visible and applied consistently. The influence of wider elements in committee deliberations should be transparent and clearly described in plain language.

Recommendations

Recommendation 26. Developing an explicit qualitative values framework

The Review recommends that the Australian Government support and resource the development of an explicit qualitative values framework by HTA advisory committees in consultation with a range of stakeholders. The framework should:

a. publish explicit guidance about the elements (beyond clinical effectiveness, cost-effectiveness and financial impact) each committee will consider, how they will consider them, and their impact on decision-making
b. allow enough flexibility for the deliberation process itself to add value to the decisions; that is, not be pre-weighted and scored
c. ensure consideration of the value elements is explicit before, during and after consideration of a technology, and transparently communicate these considerations in public summary documents
d. include documentation on how it will be considered during committee deliberations and guidance, including explaining how sponsors could provide data to respond to additional value elements and explaining how patients and citizens could provide submissions to respond to additional value elements
e. be informed by published research and public consultation
f. include a checklist to assist HTA decision-makers to integrate equity considerations into their deliberations in a more comprehensive, consistent and systematic way. The checklist should account for the fact that some new health technologies may have a negative impact on health equity. It should

also include explicit consideration of priority populations such as First Nations people

g. be consistent with Recommendation 34: Overarching principles for adopting methods in Australian HTA.

Chapter 7: Enhancing real-world data and real-world evidence for HTAs

Introduction

The health technology pipeline is dynamic – technologies evolve from concept development to clinical testing, regulatory and subsidy approval, and post-market surveillance.[140] Evidence-informed evaluations are undertaken to guide decisions at points in the pipeline, impacting the development of, and access to, health technologies.

Decision-making in the pipeline is iterative. At each point, decision-makers must assess whether the 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.[141]

As discussed in Chapter 8.3, randomised controlled trials (RCTs) are the gold standard in establishing the comparative effectiveness of health technologies, but they have limitations and in some circumstances are not feasible.[142] In an increasing number of scenarios, it's not feasible to gather sufficient RCT evidence. This is driven in part by emerging technologies that treat rare diseases and other small populations, such as those defined by precision medicine. Emerging technologies (e.g. ATMPs) also often carry a high level of uncertainty around long-term claims and late effects, which are not routinely assessed in RCTs. Further, there is increasing recognition of the importance of addressing inequities in populations not adequately represented in clinical trials (e.g. minority groups or those ineligible for trials).

This chapter covers the Review's analysis, findings and recommendations on how to establish supporting structures to facilitate timely access to best-possible, high-quality real-world evidence (RWE) for HTAs across the lifecycle including:

- developing a comprehensive data strategy and strategic oversight to optimise real-world data (RWD) and RWE for HTAs

[140] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[141] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[142] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

- developing national data infrastructure to support the generation of RWD and RWE for HTAs.

Government initiatives that are already available, such as the Medical Research Future Fund, improve the quality and availability of evidence published by external stakeholders. These initiatives can be used to support HTAs.

The most effective way to optimise the generation of evidence to support Australian HTA needs is to communicate how different types of evidence are used to inform HTA decision-making. Several of the Review's recommendations would encourage the development of fit-for-purpose evidence, and investing in developing effective communications would improve the quality of evidence submitted with evaluations, making some HTA processes more efficient. The recommendations are:

- Recommendation 24: Developing an engagement framework
- Recommendation 26: Explicit qualitative values framework
- Recommendation 33: Methods for assessing consumer evidence
- Recommendation 34: Overarching principles for adopting methods in Australian HTA
- Recommendation 35: Methods for assessing non-randomised and observational evidence.

Chapter 7.1: Optimising real-world data and real-world evidence for HTA

Introduction and context

Real-world data and real-world evidence in HTAs

RWD and RWE (generated by analysing RWD) play an important role in supporting the evidentiary needs of decision-makers across the health technology pipeline, including to inform HTAs for subsidy approvals and PMRs.

Terminology: Real-world data and real-world evidence

Real-world data is defined by the International Network of Agencies for Health Technology Assessment as data collected during the routine delivery of health care, outside clinical trial conditions. Other agencies and groups have more expansive definitions that also include data collected routinely across all aspects of health care and social care, through disease and health technology–specific registries and directly from patients through digital platforms.

Real-world evidence is the clinical evidence regarding the usage and potential benefits or risks of a health technology derived from analysis of RWD.

(*See Optimised real world evidence to support health technology assessment in Australia.*)

RWD is most commonly used to inform the PICO in HTA evaluations for subsidy approvals, to generate local inputs for cost-effectiveness modelling and to predict overall usage and costs. It is also used in the post-subsidy space to understand usage patterns and cost.

Using real-world evidence and real-world data to address uncertainties

RWE can help to resolve uncertainties in the HTA evaluation of technologies in the Australian setting. This includes assessing anticipated benefits and risks in specific populations, and long-term outcomes.

RWD and RWE have an increasing role in contributing information on the outcomes associated with funded technologies. This is particularly relevant for provisional listings with uncertainty around long-term outcomes for a specific patient population. Having systems in place to enhance the timely and accurate collection and reporting of usage and outcome data associated with provisionally listed technologies will be critical to the establishment of any MEA-type arrangements.

RWE may be used to address uncertainties in an HTA evaluation before subsidy approval to:

- estimate the comparative effectiveness of the health technology relative to existing treatments in the real-world setting, such as in scenarios outlined in Figure 5
- generate inputs for cost-effectiveness analysis by providing insights about the resource use and associated costs in the real-world setting.[143]

[143] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

Scenario	Uncertainty	How can RWE be leveraged
1. RCT evidence sufficient	Do treatments effects translate to real world	Estimate real-world safety and effectiveness Long term safety Support expanded indications
2. RCT evidence not sufficient	Comparative efficacy (surrogate end points, small sample sizes, inadequate follow up)	Confirm safety and comparative cost effectiveness in real-world settings
3. RCT evidence not available	Comparative efficacy due to lack of RCT control arm	Estimate rates of specific outcomes in usual care for nominated active comparator (as an external control arm)

Figure 5: Scenarios where RWD can generate comparative treatment effects in HTA[144]

In the post-subsidy setting, RWE can:

- support continuous assessment of use, safety, clinical effectiveness, cost-effectiveness and economic impact of health technologies in the Australian setting (which can include diverse populations, and complex, dynamic healthcare settings)
- generate input for clinical practice guidelines and benchmark guideline-recommended versus actual care
- empower patients to make informed treatment choices using the outcomes of patients with similar characteristics.[145]

Where a decision is taken to resolve uncertainty about the clinical effectiveness and/or cost-effectiveness or safety of a health technology after it has been funded, information on the health outcomes of patients receiving the health technology needs to be collected.[146]

In Australia, there are numerous instances where RWE was used to enhance and complement available RCT evidence. It has been influential in reducing uncertainty in decision-making, both at the time of initial funding decisions and post-subsidisation. This has increased timely access to therapies through the ability to monitor health outcomes after listing on the PBS. For example, several new-generation direct-acting antiviral (DAA) medicines for the treatment of chronic hepatitis C were listed on the

[144] Adapted from p8 in Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[145] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[146] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

PBS from 1 March 2016.[147] Due to the high opportunity cost, approval for listing on the PBS included allocating funding to develop a national registry for the ongoing assessment of the cost-effectiveness and health outcomes of the DAAs. At its December 2020 meeting, the PBAC considered that an economic evaluation based on the real-world registry data demonstrated that the DAA listings were cost-effective in practice and the committee was comfortable recommending not to change the PBS listings.[148]

Difficulties in timely access to outcome data associated with provisionally listed health technologies

Australia's processes and systems for collecting the information needed to determine the clinical effectiveness and cost-effectiveness of provisionally listed health technologies after they are funded are in early development.

The experience with health technologies that have been funded in Australia through MEA-type approaches is that it is difficult to establish how well the health technology is performing due to:

- challenges in establishing a suitable data collection system or involving a suitable registry
- quality and completeness of collected data
- reporting limitations, such as inadequate resources to complete data analysis within the required time frame.

There are few or no consequences for sponsors of health technologies if data is not collected as required. This is due to HTA advisory committees being limited in the advice they can provide that would revise the cost-effectiveness of the treatment in the absence of complete, high-quality and timely data. This is highlighted in the example for tisagenlecleucel (TIS).

[147] DHAC (2019) National Hepatitis C Data Collection Public Summary Document, March 2019 PBAC Meeting, PBS.

[148] See December 2020, Other matters in DHAC (2020) December 2020 *Recommendations made by the PBAC – December 2020 Intracycle meeting*, PBS.

Chimeric antigen receptor T (CAR-T) cell therapy tisagenlecleucel

The MSAC supported funding TIS in 2019 for treating acute lymphoblastic leukaemia (ALL) in paediatric and young adult patients (pALL) (*MSAC application 1748*). Public funding was supported on the basis that clinical, economic and financial uncertainty would be resolved after it was funded through:

- ongoing data collection in a registry
- a full review of clinical effectiveness, cost-effectiveness and budget impact
- a pay for performance arrangement
- financial caps.

A review by the MSAC in July 2023 found that the available evidence did not fully address the uncertainties that existed when it initially supported funding. Uncertainty about clinical outcomes beyond 12 months was not resolved due to incomplete and inadequate data.

The MSAC has since provided new advice relating to the purpose of reviews in the CAR-T cell therapy space. The PSD for *MSAC application 1723.1* (brexucabtagene for relapsed or refractory B-cell ALL (R/R B-ALL*)* noted challenges conducting a cost-effectiveness review informed by registry data to reduce the price of already funded therapies (as highlighted by the July 2023 review of TIS for pALL). It was reiterated that the Commonwealth and jurisdictional governments, along with other relevant stakeholders, need to work together to determine the most appropriate data collection mechanism for HSTs.

Quality and completeness of real-world evidence and application for HTAs

While 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 HTAs.[149]

As discussed in Chapter 8.3, the use of inappropriate statistical methods and uncertainty in the robustness of RWE, and related inadequate methodological transparency and RWE reproducibility, remain a major barrier to using RWE. This barrier can limit the ability to resolve uncertainty arising from RCT evidence, which can involve disproportionately small populations and lack generalisability to the intended treatment populations.

Data-related barriers include:

- inadequate information about the RWD to fully assess its suitability in terms of representativeness and utility

[149] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

- deficiencies in terms of data quality
- inadequate standardisation to allow comparison or integration with other sources. For example, RWD gaps prevent the accurate identification of cohorts with ultra-rare diseases and the generation of robust historical comparator groups.

The quality of RWE is multifactorial: it relies on the quality of the underlying data (provenance, reliability and completeness), the quality of the methods used to analyse the 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 and representativeness) and reliability (accuracy and completeness).

Absence of a coordinated national approach to collecting real-world data on health outcomes

A barrier to implementing MEA-type approaches is the absence of interconnected data infrastructure and standards that would enable health outcomes data to be collected and reported in a way that supports the assessment of clinical effectiveness and cost-effectiveness. These data challenges also limit the ability to conduct timely, meaningful PMR for some therapies or groups.

Although some jurisdictions have approaches for collecting health outcomes data and/or data for some disease or health technologies, often these are not coordinated or standardised. The data may not be configured in a way that would enable questions about clinical effectiveness and cost-effectiveness to be answered. Further, timely access to, and sharing of, data can be challenging due to limited resourcing and infrastructure, legislative issues, privacy and security concerns, and other administrative issues.

Legal considerations for curating and sharing data

Legal impediments to data sharing remain a primary barrier to maximising use of RWD, as some datasets cannot be legally shared to support HTAs. Examples include:

- inconsistent and conflicting data sharing legislation between the Australian and state and territory governments
- lack of clarity about the consent arrangements for sharing and reusing certain data types
- access criteria for some datasets excludes research purposes and/or support for HTAs.

Patient consent and privacy concerns

Consumer and societal concerns and lack of trust around data privacy and security arising from high-profile and impactful data breaches result in consent often not being given to collect and/or share consumer data.[150]

Data stewards reluctant to share

Data stewards (or custodians) may not be comfortable sharing data due to confusion about legislative and/or consent requirements, concerns about security and privacy, or perceived lack of control over data access and use.[151] These barriers may be addressed with co-designed guidance and governance arrangements to reduce confusion and provide assurances. Providing secure environments for users to access and analyse data, such as the Australian Bureau of Statistics (ABS) DataLab[152] approach, may also be beneficial.

Resource limitations to support data collection, curation and sharing

The high costs associated with collecting, checking and curating high-quality RWD, and lack of resources for these activities or to manage queries, often limits data collection and sharing, even where there is goodwill to do so.[153] As much of the data relevant to HTAs is initially collected for other purposes (e.g. clinical or personal records, and administrative data associated with routine patient care or social services), stakeholders wishing to use the data for an HTA often rely on what is collected by other parties (e.g. clinicians or administrators) and/or engage them in collecting data that is fit-for-purpose for an HTA. A collaborative approach to data collection to meet HTA needs, as well as those of other stakeholders, would encourage participation in relevant, standardised data collection suitable for HTAs. It would also meet other stakeholders' need to improve data relevance, coverage and quality, and reduce duplication of effort.

Lack of clarity about how real-world data and real-world evidence will be used in HTAs

Industry has indicated that uncertainty about Australian regulators and payers' evidentiary needs for RWE, and lack of guidance about how they should apply and weigh RWE in their regulatory and reimbursement submissions, does not incentivise investing in generating RWE.[154] Further, there are concerns about the risk of their commercially sensitive information entering the public domain.

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[150] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[151] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[152] Australian Bureau of Statistics (ABS) (2021) *DataLab*.

[153] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[154] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

Data sources and secondary use for HTAs

Key sources of RWD that may inform HTAs in Australia include administrative data, clinical data (e.g. electronic medical records), registries (e.g. clinical and disease registries), surveys, molecular and diagnostic data, digital technology (e.g. mobile data collected by a third party outside formal healthcare delivery), case reports and social media.[155] Each of these data sources has its benefits and limitations, but importantly, the vast majority of data used in the context of HTAs is for a secondary purpose outside the primary purpose for which it was collected.[156] It is important to note that for the majority of these sources, RWD is not collected for HTAs, so its use in HTAs constitutes a secondary purpose.[157] This can result in barriers to data sharing, as well as methodological and legal challenges.

Opportunities

With advances in technologies and tools for data collection, linkage and analysis, RWD and RWE are increasingly used to support HTA decision-making. As the capacity to link heterogeneous data at the person level has increased, it is more feasible to bring together disparate RWD collections, enhancing the uses of this data to support HTA.[158]

Australia is uniquely placed in that the Department supports and manages technologies through the lifecycle, from research funding to regulatory and subsidy approval, and post-market surveillance.[159] In contrast to the UK, Canada and the US, Australia has research, regulatory and reimbursement functions sitting under the same government department.[160]

Findings

All stakeholder cohorts supported broader use of RWE for HTAs, with the expectation that broader use and acceptance of RWE would improve overall HTA decision-making and allow more access to health technologies through standard pathways or as part of MEAs. Stakeholders consistently called for additional guidance from HTA advisory committees and healthcare payers on how RWE would be used and accepted as part of informing recommendations and decision-making.

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[155] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[156] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[157] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[158] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[159] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

[160] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

Some patient and industry stakeholders stated that existing sources of RWD should be used to generate the necessary RWE for HTA purposes. But other stakeholders in the same cohorts noted that data collection capacity and capability was limited (in terms of infrastructure and resourcing) and not necessarily fit-for-purpose, depending on the issues or uncertainties that the RWE was being used to address. To address these gaps, a number of patient, clinician and industry stakeholders suggested it would be necessary to improve the approach to collecting RWD (such as by extracting data from clinical registries and electronic health and medical records). They highlighted historically under-served sub-populations (e.g. rare diseases and First Nations sub-populations) and that emerging technologies have uncertain longer-term outcomes.[161]

These diverse views probably reflect the different perspectives of stakeholders about the quality, breadth and standardisation (linkage potential) of existing RWD sources, the utility of data for secondary purposes, and resources required for its timely collection and reporting. These stakeholder observations were similar to those expressed to The New Frontier inquiry.[162] The inquiry discussed the benefits, opportunities, risks and uncertainties associated with the consideration of RWE in HTAs. It also looked at how different use cases for RWE (e.g. as primary evidence for HTA recommendations, follow-up evidence addressing identified uncertainties, and for pharmacovigilance) can affect the design and complexity of collecting, interpreting and using evidence for subsequent decision-making.

The Review tested a number of options with stakeholders to maximise the value and availability of RWD and RWE for HTA. These received broad support. All key stakeholder groups noted that implementation would require consideration of the complexities associated with data collection within the wider health system, other applications of health data, alignment with existing initiatives, deliberate collaboration between stakeholders, and significant investment in resources to collect evidence.[163]

A small number of stakeholders at the in-person forums for Consultation 2, and some written submissions, noted that the proposed options lacked specific implementation and resourcing details.[164] However, most of these matters are covered in the supporting paper referred to in the recommendations (*Optimised real world evidence to support HTA in Australia*[165]).

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[161] See findings and observations summarised in Centre for Health Economics Research & Evaluation (2023) *Health Technology Assessment Policy and Methods Review – Consultation 1 Report*.

[162] Australian Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier Inquiry*.

[163] See Breast Cancer Network Australia, Rare Voices Australia, Antengene and Biogen submissions to Consultation 2 for representative views on these matters.

[164] Bastion Insights (2024) Consultation 2 Stakeholder Engagement Report, DHAC.

[165] Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

A number of patient and industry stakeholders noted that data security and privacy issues would need to be carefully handled to maximise patient participation in data collection and support transparency and collaboration between stakeholders.[166]

Conclusion

Given the increasing uncertainty associated with indications for emerging health technologies that meet HUCN and address the needs of disadvantaged groups, it is critical that evidence is enhanced to increase confidence in decision-making. There are significant opportunities to use RWD and RWE for these purposes. Significant investment has already been made in data collection and infrastructure, but resources and effort need to be pooled and standardised to effectively meet HTA needs. A consolidated, collaborative approach would also help to increase participation by other essential stakeholders, including clinicians, data custodians, researchers, funders and industry, by ensuring that shared infrastructure meets the purposes of different stakeholders. The roadmap set out in *Optimised real world evidence to support health technology assessment in Australia* provides a solid approach to achieving efficient, effective and coordinated infrastructure to support Australia's HTA needs.[167]

Objectives of recommendations

The Review's recommendations aim to enhance timely access to relevant, quality RWD and RWE to support:

- epidemiological modelling to understand the size and characteristics of the intended treatment population and current and intended treatment pathway, and to inform the economic model and overall costs associated with the therapy
- assessment of the comparative effectiveness of health technologies proposed for use in Australia, as a supplement to available RCT evidence, to assist with resolving uncertainty
- review of the usage and performance of health technologies in Australia to ensure that subsidised health technologies are the most appropriate, safe, clinically effective and cost-effective option for their funded indication, and potential suitability for other indications.

[166] See Consumers Health Forum of Australia and Janssen submissions to Consultation 2 for representative views on this matter.

[167] See p41–47 *'PART 2: Roadmap for optimising the availability and use of RWD to generate robust RWE to support the HTA lifecycle in Australia'*, in Centre of Research Excellence in Medicines Intelligence (2023) *Optimised real world evidence to support health technology assessment in Australia*, Health Technology Assessment Policy and Methods Review.

Recommendations

Recommendation 27. Governance and strategic oversight of real-world data to support HTAs

The Review recommends that the Australian Government develop and implement an Australia-specific framework to optimise timely access to relevant real-world data (RWD) for HTAs, to supplement available randomised controlled trial (RCT) evidence. This framework should:

a. cover enabling systems and pathways, and evaluation and research when collecting and using RWD for HTAs
b. be co-designed and developed with oversight from a multi-disciplinary, multi-stakeholder advisory group, reporting to government. It is important that the group has links to international entities and partnerships with data stewards to facilitate access to data applicable to HTAs
c. include a strategy to increase confidence, awareness and acceptance of cross-jurisdictional and cross-sectoral RWD access and use in HTAs. The strategy should:
    i. centre around consumer and community engagement and co-design, leverage and integrate existing international activities and guidelines, incorporate the Australian context and evidence, and fine-tune responses and messages specific to HTAs
    ii. support the development and enhancement of systems that ensure privacy protections, data security and First Nations data governance, aligning with existing strategies (e.g. the Department of Health and Aged Care's *Data Strategy 2022–2025*).

Recommendation 28. Data infrastructure to support HTAs

a. The Review recommends that the Australian Government develop dynamic, enduring, whole-of-government data infrastructure, with oversight by the multi-stakeholder advisory group (see 'Recommendation 27: Governance and strategic oversight of real-world data to support HTAs'). Consideration should be given to jointly funded infrastructure by the Commonwealth, state and territory governments and industry with potential access to additional stakeholders on a user-pays system.
b. This infrastructure should provide:
    i. transparent and streamlined governance
    ii. the ability to evolve over time, based on the needs of HTA agencies and other stakeholders, evolution of health and digital technologies and assessment tools, and research questions that are likely to be addressed using real-world data (RWD) and real-world evidence

iii. harmonised international standards, and be flexible and scalable, and allow transparent data quality assessment
iv. a dedicated consumer-evidence repository to collect data for future HTA activities, track expectations, and support the development of consumer-centred measurement tools
v. work towards co-designing and building an enduring, sustainable, safe, high-quality and fit-for-purpose data ecosystem that evolves over time to meet Australia's HTA needs.

c. As an immediate priority:
   i. core priority Australian RWD collections that are fit-for-purpose for HTA requirements (including registries and pharmacovigilance data) should be mapped
   ii. access should be facilitated to relevant priority RWD collections for relevant stakeholders to support HTAs
   iii. minimum data quality standards and validation and reporting processes should be implemented for priority RWD collections
   iv. Data should be harmonised across government departments and jurisdictions for government-held Australian RWD collections that are fit-for-purpose for HTAs (including data held by public hospitals and health services)
   v. RWD curation and harmonisation activities should be implemented for priority RWD collections, including internationally standardised coding and terminology.

Recommendation 29. Intergovernmental collaboration in standardised collection and sharing of health technology–related data

The Review recommends that the Australian Government promote state and territory government collaboration and participation in cross-jurisdictional data sharing to support a nationally cohesive HTA system.

This should be facilitated via centralised data-sharing infrastructure and harmonisation of access to existing government-held real-world data collections (see 'Recommendation 28. Data infrastructure to support HTAs').

Consideration should be given to strengthening and supporting the information-sharing clauses recommended in the 2020–25 Addendum to the National Health Reform Agreement, including establishment of information-sharing platform. This would help to ensure that all states and territories meet the policy and resource commitments made in relation to collecting and sharing data on the use of health technologies of interest, from pre-registration to post-market review.

Recommendation 30. Real-world data and real-world evidence methods development

The Review recommends that the Australian Government, with oversight by the multi-stakeholder advisory group (see 'Recommendation 27. Governance and strategic oversight of real-world data to support HTAs'), develop a multi-stakeholder coordinated approach to transparent evidence development for HTAs, using best-practice methods, spanning data standardisation, standardised analytics and reporting.

Recommendation 31. Collecting and using real-world data to resolve uncertainty

The Review recommends that the Australian Government ensure early identification and/or configuration of data collections potentially suitable to help resolve uncertainties (and any new randomised controlled trial evidence), where it is expected that an application is likely to result in a managed entry agreement (MEA).

a. Suitable data collections may include:
    i. priority real-world data (RWD) collections (see 'Recommendation 28. Data infrastructure to support HTAs'), such as in existing clinical registries. Integrated data from a single populous jurisdiction may be fit-for-purpose to address certain research questions)
    ii. in the longer term, outcomes of interest may be collected as add-ons to enduring data linkages, national datasets (see 'Recommendation 28. Data infrastructure to support HTAs') or electronic health records data, as recommended by the advisory group (see 'Recommendation 27: Governance and strategic oversight of real-world data to support HTAs').
b. Where a suitable data collection cannot be identified, establishing new datasets could be considered. These could be obtained via pre-agreed data items (e.g. minimum datasets) collected via relevant electronic medical records or a customised form used for collecting data as a requirement for access to provisionally listed therapies
c. Early exploration and negotiation should begin to determine the feasibility of, and resourcing requirements for, timely, quality data collection and reporting for the intended purpose
    i. Resourcing should be jointly funded by relevant parties, with fund administration and data collection overseen by the multi-stakeholder advisory group (see 'Recommendation 27: Governance and strategic oversight of real-world data to support HTAs')
    ii. Details need to be resolved before entering into any MEA, to provide assurance that uncertainties will likely be resolved via evidence generation during the provisional listing period.

d. 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
e. In the case of ultra-rare diseases and other small populations, international collaboration in the collection of patient-level data (e.g. international registries) and the potential use of common data models should be considered, where possible. Inclusion of RWD and real-world evidence obtained through local use should still serve as a mechanism to support funding suitability and clinical effectiveness, as it provides local context.

Chapter 8: Methods for confident decisions

Introduction

The decision to fund access to a health technology through one of Australia's universal access schemes is, in effect, a decision about what treatments patients will choose in certain circumstances. There is always a significant uptake of health technologies after they are funded.

These decisions, therefore, have significant implications for patients, their carers, clinicians, the health system and the taxpayer. They impact the choices made by patients and their treating health professionals. These choices in turn can have life-changing consequences for patients and their carers, and major financial consequences for the taxpayer.

It is therefore critical that when deciding to include a health technology in one of Australia's universal access schemes, decision-makers are confident that it is in the best interests of Australian citizens. That is, the health technology will perform as claimed, deliver the best possible health outcomes for Australians, and represent a worthwhile use of taxpayer funds.

This Chapter covers the methods used to support decisions to include health technologies in Australia's universal access schemes. The technical methods in guidelines for making submissions for public funding (such as the PBAC[168] and MSAC Guidelines[169]) set out the evidence and methods for evaluation decision-makers need to be confident that the health technology will perform as claimed. They are designed to help decision-makers understand:

- who should access a health technology
- whether it will work as well, or better than, existing care
- whether Australians will be better off overall if it is funded.

The methods for HTAs comprise four steps:

1. identify the PICO
2. conduct the clinical evaluation
3. conduct the economic evaluation and produce financial estimates
4. consider additional elements that may influence these steps or overall decision-making.

[168] DHAC (2016) *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*, PBS.
[169] DHAC (2021) *Guidelines for preparing assessments for the Medical Services Advisory Committee*, MSAC.

The Review identified different issues for each of these steps. These issues and the way they should be addressed are covered in this chapter.

Overall, the Review heard that the methods for assessing health technologies in Australia are very rigorous. Many participants viewed this positively because rigour is important for ensuring that patients receive the best available treatment and that funding them is not a disproportionate use of taxpayer funds. However, some participants across patient groups, clinicians and industry felt the level of rigour was too high and treatment of uncertainty in evidence was too conservative. They were concerned that this was denying access for patient populations that could potentially benefit from health technologies, or that it was leading to the latest treatments being undervalued and access delayed.

The Review also identified that emerging therapies (such as cell and gene therapies) pose different challenges compared to conventional medicines. These therapies have promise as curative treatments for previously untreatable conditions. But there are also major uncertainties about their effectiveness and the costs associated with them.

The Review found some areas where technical methods should be updated to ensure that patient populations that could benefit from treatment are not excluded and that potential benefits are captured and valued. However, it also found that a number of the concerns expressed about technical methods arose from a lack of awareness or understanding of current approaches – including those already published in guidelines and PSDs.

There was a significant lack of awareness that HTA advisory committees can and do recommend funding of health technologies when RCT evidence is not available (as is often the case for treatments for rare diseases). It is also not well understood how thoroughly HTA advisory committees consider consumer input. There are also many examples of where this has significantly impacted decision-making, including through the inputs that are used in clinical and economic evaluations. A reason for this may be that details about some of these approaches can only be found in the public summaries of HTA advisory committee decisions. They are not in guidance for sponsors or plain language explanatory materials for the public (see Chapter 6).

Accordingly, the Review received positive feedback on options for additional guidance and plain language explanations of methods, and clarification on a number of areas of technical methods.

The Review recommends updates to ensure that:

- patient populations that could potentially benefit from a health technology are identified and considered in decision-making
- sponsors and other participants in HTAs, including patients and consumers, understand what evidence and methods HTA advisory committees require to

ensure decision-making confidence, and that these requirements are provided in plain language
- guidelines address the unique methodological challenges posed by emerging therapies
- health outcomes delivered by health technologies are appropriately valued.

Chapter 8.1: Population, Intervention, Comparator, and Outcome

Introduction and context

The starting point for an HTA is defining the scope of the analysis that will compare health outcomes delivered by a health technology with the existing standard of care for a particular population. Many different frameworks are used in health care for this purpose. The main framework used to assess health technologies funded through Australia's universal access schemes is the PICO framework.

The PICO framework divides the research question into four components:

1. **P**opulation (the population being considered)
2. **I**ntervention (the proposed health technology)
3. **C**omparator(s) (the treatment or standard of care the intervention is being compared with)
4. **O**utcome(s) (the health outcomes that are measured for those who receive the intervention versus the comparator).

Different health technology advisory committees in Australia have different processes for determining the PICO. The PBAC relies on the sponsor of the health technology to describe the population to be treated, the intervention, comparator and patient-relevant clinical effectiveness and safety outcomes. By contrast, the MSAC and ATAGI seek input in the first instance from other stakeholders to ensure that the PICO reflects what is important to clinicians, patients and their carers.[170] A similar approach is used by NICE in the UK and the European Network for Health Technology Assessment.

The PICO has a major influence over who ultimately can and cannot access therapy once it is funded. By defining the scope of analysis for the HTA, the PICO effectively determines which patients the health technology will be recommended for by the HTA advisory committees. The committees have limited ability to recommend populations outside those identified by the sponsor through the PICO.

[170] Adelaide Health Technology Assessment (2023) *Determination of the Population, Intervention Comparator, and Outcome (PICO)*, Health Technology Assessment Policy and Methods Review within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

Issues

Most of the issues participants raised in the Review about identification of the PICO were about the absence of consultation with patients and clinicians during the PBAC process.

Many patient, consumer and clinician organisations felt that the absence of consultation on the PICO for PBAC submissions meant that potentially important populations that would benefit from treatment were not identified, and important patient-relevant outcomes could be missed. Some participants raised that the lack of a mechanism to compel sponsors to identify the health needs of sub-populations (such as First Nations people) created a barrier to equitable access.

Consumer and clinician organisations raised that a lack of sufficiently detailed and accessible information about the PICO for PBAC submissions meant there was often misunderstanding about the intended treatment population and the expected benefit. Industry participants expressed that the sponsor and the PBAC often disagree on key elements of the PICO, which contributes to the rate of 'not recommended' decisions and resubmissions.

What we heard:

'The RACGP welcomes the attention to increase early stakeholder input into choice of PICO (Population, Intervention, Comparison, and Outcome), as it helps prevent a product sponsor from selecting irrelevant comparators or outcome measures.'

Consultation 2 submission: Royal Australian College of General Practitioners (RACGP)

'An upfront approach – through agreement of a PICO (as already identified) and involving clinicians and patients – would assist in placing the patient at the centre of the process.'

Consultation 1 submission: UCB

'Roche notes that a PICO step should largely be optional, especially in the circumstance that the sponsor has a high level of confidence in an appropriate PICO and does not believe that the PICO scoping phase will add value that outweighs the scoping time.'

Consultation 2 submission: Roche

Most participants supported greater transparency and stakeholder engagement in identifying the PICO for PBAC submissions but felt that it should not be at the expense of timely access. A subset of participants was concerned that introducing a mandatory or structured PICO process would impact timeliness – particularly for submissions where:

- the PICO has been previously well defined
- a separate PICO process would not appreciably improve the submission development and evaluation, for commercial or regulatory reasons, and the sponsor does not wish to change its proposed PICO.

Findings

Sponsor-driven definition of the PICO for PBAC submissions excludes patients and clinicians, and results in narrower access

There is minimal opportunity for patients and clinicians to participate in the formulation of the PICO by sponsors for PBAC submissions. These groups do not have access to full information about the population, comparator and outcomes set out in submissions and have minimal opportunity to influence their selection by sponsors.

This sometimes results in the exclusion of certain populations that could benefit from access to a health technology. These populations often comprise patients with rarer variants of particular diseases or who have other characteristics that are excluded in clinical trials. This results in inequitable access, with those populations missing out on funded access to health technologies.

There is insufficient time to consult on the PICO under the existing PBAC submission time frames

The 17-week cycle for PBAC submissions leaves little time for broader consultation with patients, carers and clinicians on the PICO. Broader consultation, without impacting time frames, would need to occur before submissions to the PBAC or require a longer assessment time frame.

It is not necessary to consult on the PICO in all circumstances

Not all submissions require broader consultation with patients, carers and clinicians on the PICO. In some circumstances, the PICO is clear or has been satisfactorily established in other settings such as through horizon scanning or in overseas jurisdictions.

Conclusion

While sponsors will always have the final say over the PICO defined in their submissions, broader dialogue on the development of PICOs in PBAC submissions could make the process more patient-centred and assist appraisal of health technologies in certain circumstances. This could help to reduce inequitable access arising from exclusion of populations that could potentially benefit from therapy (such as paediatric

populations). However, consultation on PICOs should not be mandatory for all submissions. This could increase assessment time frames and would not be necessary for all submissions.

Objectives of recommendations

The Review's recommendations are intended to put patients at the centre of the development of the PICO. The recommendations intend to increase transparency for, and involvement of, patients and clinicians in the development of PICOs, particularly for submissions to the PBAC. They are intended to ensure that patient populations that could potentially benefit from access are not excluded and that outcomes that are most relevant to patients are captured. Recognising the potential for additional consultation to increase the time and complexity, the recommendations seek to ensure that consultation is used only where it is likely to facilitate decision-making in the interests of patients and avoids adding time or complexity to the HTA.

Recommendations

Recommendation 32. Creating a framework for PICO development to support HTA submissions

The Review recommends that the Australian Government:

a. work with stakeholders to establish framework principles and application criteria that govern when and how the Population, Intervention, Comparator, and Outcome (PICO) framework is to be developed in support of an HTA submission. The framework should:
    i. establish a baseline set of circumstances where a comprehensive and facilitated PICO scoping process would add value to the HTA process
    ii. ensure that any new PICO process facilitates decision-making, reduces the likelihood of resubmission churn, and avoids adding time or complexity to the HTA
    iii. ensure criteria of importance to patients and clinicians (e.g. for high added therapeutic value (HATV) that addresses high unmet clinical need (HUCN)) are appropriately considered and discussed as part of PICO development in a manner that:
        1. ensures consideration of relevant patient populations that could potentially benefit from the new therapy
        2. considers the health equity and high unmet need implications associated with access to a health technology
        3. allows discussion of issues that may affect early implementation (for new drugs or major expanded indications claiming added therapeutic value)

4. captures patient and clinician viewpoints appropriately as part of PICO confirmation outputs
5. informs the development of the HTA submission and the resulting screening, evaluation and deliberation processes by the relevant HTA advisory committee.

iv. accommodate circumstances where a level of flexibility from the normal PICO scoping and definition process may be appropriate, with clear justification. This may include situations where (for example):
   1. PICO dialogue has been conducted in alternative settings (such as via horizon scanning in stakeholder engagement processes) and information has been collected formally in a manner that serves as an appropriate functional substitute for the purposes of efficient public consultation
   2. a well-defined PICO can be adapted from a comparable overseas HTA entity and has the endorsement of key affected stakeholders to support Australian considerations.

b. establish a process with stakeholders to produce and release plain language summaries of the PICO with the Pharmaceutical Benefits Advisory Committee (PBAC) agenda to:
   i. increase transparency about the proposed treatment population and communicate the expected benefit (outcome)
   ii. assist in managing stakeholder expectations (for new drugs or major expanded indications claiming added therapeutic value).

Chapter 8.2: Use of consumer evidence and input

Introduction and context

The PBAC Guidelines and MSAC Guidelines also give applicants the option of presenting additional relevant information that may not be captured elsewhere in the assessment. This should be clearly presented and reasoned. Where possible, it should be generated using high-quality methods or sourced systematically.

Both the PBAC and the MSAC also invite comments from patients, clinicians and representative organisations, which are considered with the submission.

Consumer evidence and consumer input are two complementary but discrete approaches to informing HTAs about patients' needs, preferences, experiences and perspectives. The Review learnt that these two concepts were often misunderstood and confused at the risk of inappropriately assessing or using them. Table 3 (adapted from

Staniszewska and Werkö (2017) for Australian terms) sets out the important differences.[171]

Table 3: Distinction between consumer evidence and consumer input in HTAs

Consumer evidence (often called 'patient-based evidence')	**Consumer input (often called 'patient input')**
Produced through research, generally published in peer-reviewed journals, which may be co-designed with consumers or their organisations	Contributed by consumers and their representative organisations through engagement activities in an HTA
Draws on a range of robust methods with known strengths and limitations	Draws on lived experience of members or individuals; may include collating and reporting from discussion groups, interviews, logs and surveys undertaken by consumer organisations
Provides an appraisal of quality including formal critical assessment and peer review	Quality may depend on factors such as authenticity or diversity of perspectives
May be more limited in accounting for HTA context	Can be dynamic and more responsive to questions arising in the HTA and the local setting
Research directly addresses questions of bias and balance	Lived experience bias is part of the value, not mediated by a research perspective
Examples include qualitative evidence (including qualitative evidence synthesis), patient preference studies, patient-reported outcome measures (PROMs), patient-reported experience measures (PREMs) and patient experience data	Examples include dialogue with consumers, presentations at meetings, and written submissions collating perspectives such as consumer comments
It is sustainable, and may be used to address questions in a variety of HTAs and settings	Burden falls to consumers and their organisations

HTA advisory committees consider these additional elements to identify and report on factors that:

- are unique to the proposed technology, circumstances of use or funding arrangement that may not have been captured elsewhere

[171] Staniszewska S and Werkö S (2017) 'Patient-based evidence in HTA' in *Patient involvement in Health Technology Assessment*, K. Facey, HP Hansen and ANV Single (Eds) Springer Nature: Singapore, p:43–50, doi: https://doi.org/10.1007/978-981-10-4068-9_4.

- have considerable impact on the way the clinical and economic results are interpreted.

These factors can include PROMs and PREMs that might not otherwise be captured via standard measures of quality of life, or non-clinical outcomes for patients such as second-order effects, social benefits, carer benefits and productivity. They also include other factors that may influence decision-making such as equity, clinical need, severity, value of knowing prognosis or diagnosis, public health issues and other relevant considerations.

Consideration of consumer evidence and input is a prominent part of deliberation by HTA advisory committees in Australia. It can significantly impact the clinical and economic evaluation as well as overall decision-making.[172]

Common feedback from individuals and organisations from multiple sectors was that it was uncertain how this additional relevant evidence and input was considered in decision-making.

[172] See for example paragraph 6.46 of DHAC (2022*) Zanubrutinib Public Summary Document, March 2022 PBAC Meeting* and paragraph 7.14 of DHAC (2021) *Sacituzumab govitecan Public Summary Document, November 2021 PBAC Meeting*.

What we heard:

'People with lived experience and consumer perspectives are critical in better understanding the role and value that a medicine can bring. This is particularly so for medicines for treating people with rare diseases when little may be known or published about the condition and the researcher/developer is making available the first-ever treatment.'

Consultation 1 submission: Biogen

'Guidance and education around expectations of patient involvement in the HTA process needs to be addressed, with the Consumer Evidence and Engagement Unit working with the PBAC to provide clear guidelines for HCOs on the types of information that is valued and the standard of evidence that can support evaluation, so that patients can meaningfully participate.'

Consultation 1 submission: AstraZeneca

'This is critical and has to be recognised as such. The education and agreement of all stakeholders in how this evidence is weighted and how it is utilised must be transparent and reported on. Best practice models should also be developed to inform all those gathering consumer evidence.'

Consultation 2 submission: Genetic Support Network of Victoria

Findings

How consumer evidence and input is considered by HTA advisory committees is uncertain

As discussed in Chapter 6, this uncertainty is partly due to a lack of information about how consumer evidence and input should be prepared and presented, in sponsor submissions and consumer comments. It was perceived that this lack of information impeded bringing a strong and useful consumer perspective into the decision-making process, and resulted in it only being used in appraisals.

Additionally, the outcome measures used in HTAs can sometimes be contentious or not representative of particular patient populations. While patient-relevant outcomes (PROs) are generally considered in the form of quality-of-life ratings, the relevance of particular outcome measures can vary for different groups, and sometimes commonly used quality of life tools use domains that are less relevant for populations with different lived experience to the developers of the tools (e.g. people living with life-long, degenerative conditions and First Nations people). Additionally, sometimes PROs

derived from the published literature may be clinician reported, rather than being based on those living with the condition or experiencing the treatment.

Therefore, many stakeholders are calling for the greater inclusion of PROs in clinical trials and the use of validated disease-specific PROMs when necessary to ensure that outcomes being measured are meaningful to the population or populations under consideration.

Conclusion

Consumer evidence and input are considered extensively by HTA advisory committees, but their impact on decision-making is not understood from PSDs, and appropriate methods and use are not clear in the guidelines. This has created uncertainty for sponsors, clinicians and consumers about how to prepare and use this evidence and input, and the impact it has on decision-making. In turn, it has, at times, impeded the inclusion of a useful patient perspective in the decision-making process.

Objectives of recommendations

The Review's recommendations are intended to ensure that sponsors and consumers understand how to prepare consumer evidence and input about patient experiences and perspectives so that it can inform the HTA decision-making process.

Recommendation

Recommendation 33. Methods for assessing consumer evidence

The Review recommends that the Australian Government support the development of updates to the Pharmaceutical Benefits Advisory Committee (PBAC) and Medical Services Advisory Committee (MSAC) Guidelines, assessment methods, public summaries and other explanatory materials to ensure it is clear how both consumer evidence (research into patients' needs, preferences, experiences and perspectives) and consumer input arising from engagement processes (see Chapter 6) may be integrated into HTAs. The updates should:

a. include further guidance on the preparation, use and evaluation of consumer evidence and input, and evidence about equity in submissions. This should include guidance on use of:
    i. qualitative evidence
    ii. patient-reported outcome measures
    iii. patient preference studies
    iv. patient-reported experience measures
    v. evidence about why patients and clinicians in Australia want or need a substitute for current care
    vi. consumer input or use of consumer evidence in R&D relating to the product.

b. clarify how Population, Intervention, Comparator, and Outcome (PICO) scoping; and clinical and economic evaluation steps can be informed by this evidence
c. elicit evidence that would help determine applicability to the Australian setting such as:
    i. evidence from relevant Australian trials (if any), including any evidence specific to Australia in terms of comparative effectiveness and safety and/or patient preferences to inform the clinical evaluation
    ii. evidence from Australian patients about their experiences using the technology, including where patients see its use in Australian practice.

Chapter 8.3: Clinical evaluation

Introduction and context

The PBAC Guidelines and the MSAC Guidelines instruct applicants to present the best available clinical evidence to support the effectiveness and safety of the proposed medicine and patient indication.

To make the best choice about treatment, patients, clinicians, HTA advisory committees and funders must understand the available evidence about how safe and effective a health technology is compared to alternative treatments patients would use.

The clinical evaluation in submissions to HTA advisory committees serves this purpose by evaluating the best available evidence and providing a conclusion about the effectiveness and safety of a health technology relative to the chosen comparator.

This conclusion supports decisions about the circumstances under which access to a health technology should be funded, and whether any additional cost is commensurate with any improvement in outcomes for patients.

Preferred evidence and constraints in the evidence base

Throughout the Review, there was an often-repeated claim that HTA processes in Australia do not recognise constraints on evidence that can be generated for certain health technologies, such as those that treat rare diseases. This is not accurate. Constraints in the available evidence base (such as for health technologies that treat rare diseases and advanced therapies) are recognised.

While the PBAC Guidelines[173] and the MSAC Guidelines[174] state a preference for clinical and economic evaluations to be based on direct RCTs, they also provide guidance on including non-traditional evidence such as indirect comparisons, non-randomised studies and non–peer reviewed studies (e.g. clinical study reports). The approaches

[173] DHAC (2016) *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*, PBS.
[174] DHAC (2021) *Guidelines for preparing assessments for the Medical Services Advisory Committee*, MSAC.

used in Australia are similar to those of other comparable countries, and consistent with best practice.[175]

Numerous examples can be found in PSDs of health technologies being recommended for funding by HTA advisory committees in the absence of direct RCT evidence, and where they instead relied on observational evidence (including RWD or RWE).[176]

Why are randomised controlled trials preferred over other types of evidence?

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

For RCTs, blinding and randomisation controls are used to ensure that bias and confounding is minimised, data is 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, can cause investigators to incorrectly conclude that differences in health outcomes between treated and untreated study participants are due to the intervention.

In contrast, studies using observational evidence, 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 observational evidence is multifactorial: it relies on the quality of the underlying data (provenance, reliability and whether data is missing), the quality of the methods used to analyse the data (appropriate study design and analytic methods to control for bias) and the quality of the question itself (data being fit-for-purpose to address the question).

RCTs, therefore, allow decision-makers to have greater confidence that the health technologies they decide to fund will perform as claimed.

Changing evidence landscape

The Review heard from a range of participants that emerging therapies (such as cell and gene therapies, and other therapies that target biomarkers) pose different

[175] Adelaide Health Technology Assessment (2023) *Clinical Evaluation Methods in HTA*, Health Technology Assessment Policy and Methods Review within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

[176] See for example DHAC (2022) *Cemiplimab Public Summary Document, March 2022 PBAC Meeting*, PBS.

evidentiary challenges to conventional medicines. Patients, clinicians and industry called for greater 'recognition and acceptance' of RWE and RWD, particularly for therapies where it is difficult to undertake RCTs (such as those for small patient populations).[177]

The Review also heard that methods for assessing non-traditional non-RCT evidence are becoming more sophisticated. Additionally, methods for controlling for potential bias and confounding in these studies are being developed globally, improving the confidence in conclusions made using these datasets.

Participants in the Review expressed a desire for greater guidance on preparing submissions for emerging therapies and using more advanced methods for assessing non-RCT evidence.

Instructions on how to implement methods in guidelines

The Review heard that there was a lack of guidance on how to implement methods in guidelines.

Most participants agreed that additional guidance in several areas of clinical evaluation would help to improve the quality of submissions and consumer input to advisory committees, including on:

- the use of non-randomised and observational evidence (including RWD and RWE)
- the use of surrogate end points
- assessment of consumer input
- methods preferred by decision-makers
- therapies that target biomarkers.

Participants generally supported proposals to generate a curated list of methodologies and update methods for assessing non-randomised and observational evidence and surrogate end points. These were particularly supported by the pharmaceutical industry, which considered these would assist sponsors in developing HTA submissions in areas with evidentiary deficiencies.[178] Pharmaceutical companies also expressed some caution about how prescriptive guidance should be, and that flexibility should be maintained. All participants felt that industry, clinicians, patients and consumers should be included in their development.

[177] See Pfizer's submission to Consultation 2.

[178] See Roche's submission to Consultation 2.

Findings

Clinical evaluations in HTAs, and the evidence that supports them, are critical to determining best treatment options for patients

In clinical evaluations, comparison of health outcomes delivered by new health technologies with those of alternatives treatments is fundamental to patients receiving the best available treatment for their circumstances.

Assessments of the safety and effectiveness of a health technology compared to alternative treatments depend on the evidence presented in submissions to advisory committees. A patient, a clinician, an HTA advisory committee or a funder can only be as confident that a health technology is the best treatment option for a patient as the evidence for it allows.

The more elements of uncertainty in the evidence (such as confounding and bias), the more difficult it is for decision-makers to be confident a health technology will perform as claimed, and for patients and clinicians to make informed choices. To that end, expectations about, and grading of, the quality of evidence presented in submissions are critical for determining the best treatment for patients in particular circumstances.

The HTA system in Australia already recognises constraints on available evidence for certain health technologies and considers real-world data and real-world evidence when included in submissions

Some stakeholders told the Review the existing preference for the best available evidence, in particular RCT evidence, disadvantages health technologies intended to treat rarer conditions where RCT evidence is less likely to be available. Many stakeholders advocated for greater recognition and acceptance of RWD and RWE.

The Review found that the preference for the best available evidence, such as RCT evidence, has not precluded consideration of other evidence where RCTs are not feasible. The ability to present non-RCT evidence, where RCT evidence is not feasible, is already built into the PBAC Guidelines[179] and the MSAC Guidelines.[180] As referenced earlier, these committees have and will make decisions based on non-RCT evidence when it is recognised as the best available.

RWE and RWD is also regularly used in HTAs for a range of purposes. In most jurisdictions, RWE is successfully used to help determine:

- the appropriate comparator
- the natural history of the disease
- treatment pathways
- long-term side effects

[179] DHAC (2016) *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*.

[180] DHAC (2021) *Guidelines for preparing assessments for the Medical Services Advisory Committee*.

- resource use
- incidence
- compliance
- quality of life
- some parameters for economic analysis.

In most cases, these uses of RWE are well established and accepted. However, RWE is used less frequently for establishing treatment effectiveness. This is due to concerns about:

- data quality and acceptability
- bias and confounding
- lack of training in HTA and methods for evaluation
- trust and transparency
- lack of standardisation
- transferability.

When RCT evidence is not available, evidence of treatment effectiveness is more uncertain. This has flow-on impacts on the confidence that advisory committees and decisions-makers have about the claimed clinical benefits of a health technology.

More guidance is needed on methods in areas where the evidence base is changing

Two primary drivers are changing the evidence base and guidance on their use in HTAs is limited.

The first driver is the increasing number of health technologies (such as cell and gene technologies) that target biomarkers and/or treat rare diseases. They are evidenced by trials with small sample sizes or that lack controls used in RCTs, involve greater assumptions about long-term benefits, sometimes carry high risk of adverse events or death, require complex implementation, and can cost several million dollars for a course of treatment for a single patient.[181]

The second driver is the increasing sophistication of methods for assessing and linking RWD.

Existing guidelines do not give participants enough clarity on the HTA process for presenting, using and assessing emerging evidence, and evidence for emerging technologies.

[181] DHAC (2024) *Emerging health technologies*.

Guidelines do not provide detailed advice on how to implement and apply many of the methods they describe

In jurisdictions such as England, detailed technical support documents have been developed to assist sponsors and evaluators to determine how to implement methods set out in guidelines.[182] The absence of such documents in Australia may contribute to a perception that certain types of evidence (such as RWE, RWD or surrogate outcomes) are not considered or accepted or that there is not flexibility where generation of higher quality evidence, such as RCTs, is not feasible. It may also contribute to the number of submissions that are too unreliable to be used for decision-making.

Conclusion

Given the importance and major public impacts of decisions about funding for new health technologies, decision-makers need to be confident they will perform as claimed. HTAs play an important role in incentivising the generation of evidence that enables decision-makers to make these decisions confidently. To those ends, it is vital to maintain preference for the best possible evidence to inform clinical evaluations.

Methods nevertheless need to adapt to the changing evidence base. The increasing number of treatments for rarer conditions (such as cell and gene therapies) and for targeting biomarkers will not be supported by evidence that delivers the same confidence as evidence that supported emerging technologies in the 1990s and 2000s.

Objectives of recommendations

The Review seeks to ensure that decision-makers have the best possible evidence available when deciding whether to recommend funding of health technologies through Australia's universal access schemes. To that end, recommendations propose further and updated guidance to ensure that sponsors understand HTA advisory committees' preferred methodologies and approaches. This includes for use of non-randomised and observational evidence, surrogate end points and therapies that target biomarkers. The Review also seeks to ensure guideline updates, and additional guidance, continue to adhere to principles that require evidence to be sufficiently rigorous to support confidence in decision-making.

[182] See p363 in Adelaide Health Technology Assessment (2023) *Clinical Evaluation Methods in HTA*, Health Technology Assessment Policy and Methods Review within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

Recommendations

Recommendation 34. Overarching principles for adopting methods in Australian HTAs

The Review recommends that the Australian Government adopt overarching principles for the methods used in HTAs for decision-making about reimbursements. These should include:

a. maintaining preference for:
    i. the best available evidence
    ii. methods that are fit-for-purpose, transparent and only as complex as required to address the problem
    iii. justification of the use of more complex methods.
b. greater acceptance of uncertainty and complex methods where:
    i. managed entry agreements are proposed, and/or
    ii. a health technology is likely to provide high added therapeutic value in areas of high unmet clinical need (HUCN).
c. provision of:
    i. guidance on methodologies preferred by decision-makers
    ii. training and guidance for evaluation groups on new methods
    iii. feedback to sponsors on their use and presentation of analysis based on more complex methods.
d. consultation with stakeholders on adoption of methodologies.

Recommendation 35. Methods for assessing non-randomised and observational evidence

The Review recommends that the Australian Government support updates to methods, in consultation with stakeholders, for using non-randomised and observational evidence, in line with the overarching principles for adopting methods in HTAs.

Revised methods should include the following updates:

a. use of indirect comparisons to include the presentation of a comparison of study characteristics, as well as how successful efforts for controlling for differences in characteristics are likely to be
b. creation of control groups to include:
    i. justification of why an indirect comparison is not possible, or less reliable, than the proposed approach of creating a control group
    ii. justification for using methods that are not pre-specified in the study protocol of the proposed technology
    iii. multiple approaches and/or multiple data sources, if possible, and a discussion of any inconsistencies in estimates.
c. use of non-randomised studies to estimate a treatment effect only where:

i. well justified
ii. design and analysis elements are sufficiently rigorous to support confidence in decision-making such as through:
    1. prospective design (preferably in collaboration with an HTA or regulatory scientific advice)
    2. registration and transparent reporting
    3. inclusion of multiple sensitivity analyses demonstrating consistency of effect.

d. adjustment of the treatment effect in the presence of treatment switching to include:
    i. multiple methods to be reported to show consistent results. This may include alternative approaches (not only methods to adjust for treatment switching) such as translating intermediate end points unaffected by treatment switching into final outcomes
    ii. a justification of the use of methods that are not pre-specified in the trial protocol of the key study for the proposed technology.

Revised methods should include more guidance on using real-world data (RWD) and real-world evidence (RWE) including:

a. for the data sources that would be acceptable for particular purposes (e.g. costs, utilities and treatment effect)
b. for assessment of the quality of the data source.

Revised methods should ensure RWE is used to determine treatment effectiveness only where the following conditions are met (or there is a strong justification that they cannot be met):

a. the technology is for use in areas of high unmet clinical need (HUCN)
b. higher-quality evidence cannot be generated or will not be generated in a timely fashion
c. limitations of RWE and impact on decision-making confidence are mitigated such as through:
    i. presentation of multiple sources of RWE (including methods of generating RWE from a source, and from multiple RWD sources)
    ii. pre-specification of the use of RWE is in the study protocol for the proposed technology.

Consideration should also be given to setting minimum standards of data quality before data is used in an HTA.

Recommendation 36. Methods for assessing surrogate end points

The Review recommends that the Australian Government support the development of additional methods for using surrogate end points in HTAs, in line with the overarching principles. This guidance should:

a. include circumstances where surrogates would be acceptable (and may include a list of previously accepted surrogate end points paired with use cases)
b. provide further instruction on evaluating evidence using surrogate end points, including 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).

Development of additional methods should include examination of methods required to validate surrogates to ensure they are consistent with methods used internationally. They should also include methods for describing uncertainty, particularly where surrogate relationships are used in combination with other methods (such as indirect comparisons or model extrapolation) where uncertainty may be substantially increased.

Recommendation 37. Methods preferred by decision-makers

The Review recommends that the Australian Government:

a. support the generation of a curated list of methodologies that are preferred by decision-makers, in collaboration with evaluation groups and sponsors. The list should include methodologies for the appropriate use and assessment of consumer evidence, real-world data (RWD) and real-world evidence (RWE). For each method in the list, brief guidance should be created that includes:
    i. a description of the method including links to key peer-reviewed articles
    ii. 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
    iii. 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
    iv. a brief explanation of how to interpret the results derived by a method
    v. a brief lay explanation of the method for the benefit of patients, clinicians and the broader public to be incorporated into plain language explanation of Pharmaceutical Benefits Advisory Committee (PBAC) and Medical Services Advisory Committee (MSAC) Guidelines.
b. support further training and guidance for evaluation groups when adopting new methods
c. support the provision of feedback to sponsors on their use and presentation of analysis based on more complex methods, and continue this practice for existing pre-submission advice, commentaries, advisory sub-committee and committee advice, or as revised as part of the Review.

Recommendation 38. Therapies that target biomarkers (e.g. tumour-agnostic cancer therapies and therapies that target cells with particular gene alterations)

The Review recommends that the Australian Government:

a. support the development of further guidance on methods for assessing tumour-agnostic therapies informed by:
    i. approaches that have been used by the Pharmaceutical Benefits Advisory Committee (PBAC)
    ii. models proposed in academic literature
    iii. models adopted in other jurisdictions
    iv. consultation with patients, clinicians and industry.
b. support the development of guidance on the assessment and appraisal of genomic technologies and gene therapies for HTA decisions in Australia.

This could be for gene therapies only if PBAC's remit remains as appraising medicines, vaccines, advanced therapies and codependent technologies. Alternatively, if the unified HTA pathway (see 'Recommendation 4. Unified HTA pathway and committee approach for all Australian Government funding of health technologies') is adopted and a single HTA advisory committee is constituted in Australia, it would include companion genomic and pharmacogenomic tests more generally (i.e. for funding decisions for all associated technologies).

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 stakeholder consultation with patients, clinicians and industry, but also people who do not have an immediate vested interest in these technologies.

Chapter 8.4: Economic evaluation

Introduction and context

The PBAC Guidelines 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.

In Australia, economic evaluations support the Government's Fiscal Strategy objective of improving living standards for all Australians.[183] They are a tool 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

[183] See p87 in Australian Government (2023) *Budget Strategy and Outlook Budget Paper No. 1*.

management.[184] In particular, they help ministers and entities uphold the principle that all expenditure should constitute proper use and management of public funds, and the most efficient, effective, economic and ethical way to achieve the maximum economic benefit for Australians.[185]

Approach for HTAs in Australia

When providing advice that supports a funding decision for a health technology that costs more than alternative therapies, HTA advisory committees need to be satisfied that the health technology 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 NHA.

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

The economic evaluation is the tool 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 the health technology provides over alternative therapies.

Economic evaluations are a common feature of government priority setting globally, including in HTAs. The paper *HTA Methods: Economic evaluation* 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.[186]

In practice, economic evaluations in HTAs:

- 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.

[184] Australian Government, Department of Finance (2022) *Budget Process Operational Rules (BPORs)*.

[185] Australian Government, Department of Finance (2022) *Budget Process Operational Rules (BPORs)*.

[186] See p5 in Centre for Health Economics Research & Evaluation (2023) *HTA Methods: Economic evaluation*, Health Technology Assessment Policy and Methods Review.

Issues

Participants in the Review across industry, patient organisations, research and clinical practice sectors perceived certain features of the PBAC and the MSAC's economic evaluation methods were 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 health care in the base case economic evaluation (e.g. second-order effects, social benefits, carer benefits and 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
- the absence of an explicit framework incorporating equity considerations into conclusions about whether cost-effectiveness was acceptable.

Stakeholders perceived that undervaluing 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.

Findings

Some features of economic evaluation cause estimates of cost-effectiveness to vary for reasons unrelated to price and benefits

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

- new health technologies where the comparator is either an old standard of care (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.

Valuing long-term benefits

Discounting of costs and benefits each year they accrue, and approaches for assessing uncertainty, mean costs and benefits are less valued the further into the future they are realised. This reflects the assumed societal preference for current over future benefits, and 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 less valued. 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). However, 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 two 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 that spread more evenly over the period in which benefits accrue into the future, notwithstanding equivalent overall costs and health outcomes.

The flexibility afforded by the absence of an incremental cost-effectiveness ratio (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 the ICER for the meningococcal B vaccine for Aboriginal and Torres Strait Islander children was acceptable when costs and benefits were discounted at 3.5% per year instead of the base case rate of 5%.[187]

There is no explicit guidance on how this flexibility is exercised in PBAC Guidelines[188] or MSAC Guidelines.[189] The Guidelines do not state that this flexibility may be exercised, particularly for health technologies that deliver long-term benefits and have upfront costs. This contributes to a perception that these technologies are valued less than those that have costs that are spread over the period that benefits accrue. This issue is not unique to HTAs in Australia. The literature review on economic evaluation found that approaches to valuing future costs and benefits in Australia are not significantly

[187] DHAC (2019) *Multicomponent Meningococcal group B vaccine Public Summary Document, November 2019 PBAC Meeting*, PBS.

[188] DHAC (2016) *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*, PBS.

[189] DHAC (2021) *Guidelines for preparing assessments for the Medical Services Advisory Committee*, MSAC.

different to those used in other countries, although Australia's discount rate is at the upper end of those in comparable jurisdictions.

The Review did not receive evidence of specific company decisions to not bring health technologies to Australia because of the base case discount rate. However, 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 less cost-effective, may be perceived as undervaluing these technologies.

Low-cost comparator (where health technologies are claimed to be 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 compared to 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 a more expensive comparator – even where the price and the number of QALYs gained are equal for both health technologies.

For therapies where it has been demonstrated they offer an improvement over the old standard of care 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 a placebo or SOC.

There is no explicit guidance on how this flexibility is exercised in the PBAC or the MSAC Guidelines. This contributes to a perception that health technologies that deliver small incremental benefits over a cheap SOC are less likely to have acceptable cost-effectiveness compared to an expensive SOC.

There is a lack of guidance on the circumstances where a technology claimed to be non-inferior can cost more than a lower-cost alternative.

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. For the PBS, this requirement is set out in section 101(3B) of the NHA.

The operation of section 101(3B) of the National Health Act 1953

Whenever it is assessing an application for a new medicine, section 101(3B) requires the PBAC to consider:

a. what are the alternative therapies to the new medicine
b. whether the new medicine is substantially more costly than an alternative therapy or therapies
c. if so, whether it is significantly more effective, or safer.

The PBAC can consider the lowest cost alternative directly or indirectly. An example of an indirect method 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 safer 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.

This requirement has been interpreted as a requirement that sponsors must select the lowest cost alternative as the comparator in submissions. This is not accurate. The fundamental question in determining an appropriate comparator for a submission is which therapy is most likely to be replaced in clinical practice.

The impact of this requirement is that a different alternative therapy to the main comparator (sometimes referred to as a 'cost comparator') may be required to be used to determine the appropriate price and cost. This occurs where the main comparator is not priced efficiently. Australia does not have policies that encourage the use of established medicines that remain comparatively effective and safe as more recently listed alternatives, with lower prices. This results in erosion of the market of older and lower-priced medicines.

The Review also heard that this requirement meant that health technologies had to be funded at the cost of inferior, or clinically irrelevant, therapies. This is also not accurate. A health technology that is claimed to be non-inferior to the main comparator can cost more than other lower-cost alternative therapies if the PBAC is satisfied that the lower-cost alternative therapies are, for compelling clinical reasons, no longer accepted in clinical practice, or for some patients have significantly inferior safety and efficacy.

This requirement has become controversial for PBS listings, in particular where the supplier of the health technology expects that it will be funded at the price of the selected comparator 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
- the advisory committee considers a cheaper therapy that is not the comparator chosen by the supplier is a relevant alternative therapy (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. 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 (not limited to clinical trials, noting that clinical trial design requirements do not address these questions). The Review also heard that in some circumstances, sponsors may not initially choose to 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.

Several participants in the Review expressed a view that it should be assumed that therapies with a very low market share should be considered irrelevant and excluded. However, the Review also heard that in Australia the market share for drugs that are no worse than newer ones is often declining for no obvious reason, hence reliance on market share arguments alone is insufficient. There must be a corresponding clinical rationale that would allow the PBAC to be properly satisfied that there is some advantage over the lower-cost alternative.

The PBAC can take these factors into account when deciding if a health technology can cost more than a lower-cost alternative. However, its guidelines do not set out the types of evidence it would need to satisfy itself that a health technology claiming non-inferiority to the main comparator can cost more than a lower-cost alternative.

Uncertainty about clinical effectiveness and cost-effectiveness is one of the main reasons submissions are not accepted and methods for managing uncertainty after funding are underused

Uncertainty about estimates of clinical effectiveness, cost-effectiveness or cost are often cited as concerns 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.

Confidence in estimates can often 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 is being used as a tool for 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 advisory committees in their decision-making and 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 advisory 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 effectiveness or cost-effectiveness of a health technology, one option for HTA advisory committees is to advise implementing a managed entry scheme or other performance-based arrangement that allows resolution of uncertainty after funding. To date, such arrangements have been implemented infrequently and only in special circumstances where there is HUCN and the health technology has HATV (see Chapter 4).

Economic evaluations in HTAs serve two competing objectives – valuing and price setting

Like many other Government investments, health interventions deliver a return to 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 *HTA Methods: Economic evaluation*[190] found that the use of cost-utility analysis and cost-effectiveness analysis (estimating 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 analysis, which is 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 HTAs in Australia and elsewhere may reflect the fact that economic evaluations in HTAs serve dual purposes – estimating value and determining the appropriate price to be paid. Ordinarily, the value expected to be returned from an investment should be greater than the price paid for it.

[190] Centre for Health Economics Research & Evaluation (2023) *HTA Methods: Economic evaluation*, Health Technology Assessment Policy and Methods Review.

Accordingly, for most government investments, the value 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, as well as the cost of production. These processes help to ensure that the overall benefit from funding an investment is shared between the public (through the return on investment of net welfare gains) and the producer (return on investment for development and profits).

A value-based approach to setting prices is necessary in HTAs because HTA bodies and payer agencies do not have access to information that would enable them to calculate the cost of production. Even if they did, they would have limited ability to obtain prices by reference to the 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 determinant 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 funds. This would reduce the net welfare gain to society when public funds are used for health technologies and increase producer profit.

If the price reflects the maximal value of the health technology to the patient and society, the welfare gains to society from funding the health technology are lost. The relative value for money of funding health technologies would then be less, compared to other potential investments of public funds 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 funds 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). Adjusting economic evaluation parameters to pay more 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.

The Review heard that the welfare loss from delayed funding of health technologies should also be considered. The Review seeks to address this issue through recommendations for bridging funding, faster resolution of price negotiations, and increased use of MEAs. It does not seek to address this issue through recommendations that HTA advisory committees accept health technologies are cost-effective at higher prices than they do now.

Acceptance of higher prices upon funding of a technology results in paying higher prices over the lifetime of the technology. Further research is needed to ascertain to what extent, and under what circumstances, Australian citizens would accept paying higher prices for health technologies compared to now. While there is a welfare loss from not funding health technologies, or not funding them until an acceptable price is offered, committing to higher overall costs for the lifetime of health technologies would significantly increase welfare losses arising from other displaced investments that would also have enhanced community welfare.

The listing of medicines on the PBS already receives favourable treatment under budget rules that otherwise require the cost of new expenditure proposals to be fully offset by direct savings to other parts of the budget. Over the past decade, successive governments have committed to list on the PBS all new medicines recommended by the PBAC. 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. This, in turn, ensures a significant (although unquantified) return on investment in terms of welfare gain for society.

Economic evaluation is being used as a tool for price negotiation

Due to its price-setting function, 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.

HTA advisory committees' 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 HTA advisory committees as offering improved efficacy or reduced toxicity over all alternatives are almost never recommended for listing on the first submission.[191] 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 sponsors expect will give them the highest levels of earnings, with high levels of return on investment.

[191] See p37 DHAC (2023) *Australian market authorisation, funding and assessment pathways and timelines*, Health Technology Assessment Policy and Methods Review.

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

Conclusion

Some limitations in economic evaluation tools cause estimates of cost-effectiveness to vary for reasons unrelated to price and benefits. But it is clear that the existing flexibility of the HTA advisory committees to interpret economic evaluations allows for appropriate recognition of value in most circumstances. However, the PBAC and MSAC Guidelines do not make it clear when HTA advisory committees may consider varying approaches, such as where they consider lower base case discount rates for health technologies with upfront costs and benefits that accrue over a long period of time.

As long as economic evaluations in HTAs have a price-setting function, they will be used as the proxy for price negotiation, and there will be a tension between recognition of appropriate value and a fair price. It is not 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 HTA advisory committees should recommend funding irrespective of how optimistic assumptions are or how cost-ineffective initial prices would be.

The Review's recommendations allow resolution of issues over a shorter time frame (see Chapter 4) and more effective management of uncertainty after funding (see Chapter 5). This is more feasible than options that would force the supplier or funder to accept funding conditions that they would otherwise consider unacceptable.

While the Review heard from several participants, including patients, clinicians and industry representatives, that the broader value of health technologies should be recognised in economic evaluations, further research is needed to determine the circumstances in which Australian citizens should accept higher prices.

Objectives of recommendations

The Review seeks to ensure that economic evaluation methods do not cause estimates of cost-effectiveness to vary inappropriately. It also seeks to ensure that sponsors understand the types of evidence required to satisfy the PBAC that health technologies claiming non-inferiority to the main comparator can cost more than other lower-cost alternative therapies. To the extent that updates to the PBAC and MSAC Guidelines have downstream financial impacts, these will need to be costed and considered in a budget context before being implemented. The Review also seeks to ensure that Australia's HTA processes are informed by Australian citizens' expectations for pricing of new health technologies.

Recommendations

Recommendation 39. Discount rate

The Review recommends that the Australian Government:

a. support a reduction of the base case discount rate to no lower than 3.5% for health technologies with upfront costs and benefits that are claimed to accrue over a long period (such as gene therapies and some vaccines)
b. determine the base case discount rate for those health technologies as part of its consideration of the financial impacts of implementing the Review's recommendations.
   Note: reducing the discount rate will result in greater attribution of value to future benefits, and may increase the need for performance-based mechanisms that satisfy the HTA advisory committee that uncertainty about future benefits, long-term safety, estimates of cost-effectiveness, and overall cost, will be effectively managed for the period that benefits are claimed.

Comment by Ms Elizabeth de Somer, Member Nominated by Medicines Australia: 'The industry recognises the movement in the discount rate in the recommendation and maintains that the base case discount rate should be reduced to 3.5% for all health technologies and 1.5% for those medicines where the benefits accrue over a longer time.'

Recommendation 40. Comparator selection

The Review recommends that the Australian Government support updates to the Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines to clarify what alternative therapy should be selected as the main comparator in submissions for health technologies with multiple alternative therapies.

For health technologies that sponsors claim are non-inferior to the selected comparator, updates should make clear that they can cost more than other lower-cost alternatives if the PBAC is satisfied that those lower-cost alternatives:

a. are for compelling clinical reasons, no longer accepted in clinical practice as alternative therapies, or
b. have, for some patients, significantly inferior safety or efficacy.

Updates should provide guidance on the types of evidence the PBAC requires to satisfy itself of these conclusions. This could include:

a. evidence from studies that the health technology provides clinical or other benefits that would significantly improve health outcomes for at least some patients compared with lower-cost alternatives
b. whether the health technology has a different mechanism of action, or other differences, compared to all existing alternatives and there is evidence that significant improvements to health outcomes would be achieved by giving

patients and clinicians the choice of multiple different alternative therapies (enabling switching in the event of treatment resistance, failure or intolerance)

c. the extent of use of the lower-cost alternative in contemporary clinical practice, supported by clinical rationale for lower use.

Before updating its Guidelines, the PBAC should seek input from patients, clinicians and industry to identify the types of evidence that sponsors should be instructed may be relevant to the PBAC's consideration. The PBAC should consider the appropriateness of any suggestions before incorporating them into its Guidelines.

Comment by Elizabeth de Somer, Member Nominated by Medicines Australia. 'The industry recognises the importance of the updated PBAC Guidelines that provide clarity to the PBAC and maintains this would be strengthened with an alternative recommendation:

The National Health Act includes an additional clause to clarify that, in subsections 101(3A) and (3B), in having regard to the alternative therapy or therapies for the relevant patient population and any sub-populations, the Committee must consider the therapy or therapies most likely to be replaced in clinical practice.'

Recommendation 41. Cost-minimisation submissions

The Review recommends that the Australian Government investigate mechanisms to differentiate cost-minimisation submissions based on their proportionate benefit and relative cost in line with other options in the Review to calibrate the methods and level of appraisal to the level of risk and clinical need and/or benefit of submissions.

Recommendation 42. Valuing and pricing

The Review recommends that the Australian Government conduct research to understand if and when it may be reasonable for HTA advisory committees to accept higher prices for health technologies than are currently accepted. This includes:

a. in what circumstances
b. for what benefit
c. how much greater cost would be reasonable to secure the benefit
d. the level of confidence needed that the benefit would be secured
e. measures that would be appropriate to offset the higher costs over a product's lifecycle.

To ensure the sentiment captured through workshops and consultations is representative of the Australian population, they should include a population representative sample (including representatives of key stakeholder groups) and ensure measurement is free from selection bias.

Note: workshops could also be assisted through the use of the explicit qualitative value framework proposed above (see 'Recommendation 26: Developing an explicit qualitative values framework')).

Chapter 8.5: Environmental considerations

Introduction and context

Globally, health care 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. In relation to Scope 1[192] and Scope 2[193] 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 underway 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 lifecycle assessment'. This maps environmental impacts associated with each stage of a product's lifecycle, including raw material extraction, manufacturing and assembly, use, and end of life ('cradle to grave'). Environmentally extended input-output analysis, an alternative method that uses only financial data to estimate footprints, is not appropriate for this purpose.

The Department launched its National Health and Climate Strategy, Australia's first such strategy, in December 2023.[194] It outlines priorities for the next 5 years to address the health and wellbeing impacts of climate change. It sets out actions that will build healthy, climate-resilient communities, and a sustainable, resilient, high-quality, net zero health system. There was broad stakeholder support for including the environmental impacts assessments being considered in HTA processes.

Findings

Through Consultation 2, including its face-to-face meetings, the Reference Committee heard evidence that the majority (60%) of healthcare greenhouse gas emissions were associated with providing clinical care, and therefore driven by clinical decision-

[192] Scope 1 emissions (direct emissions) are produced from sources within the boundary of an organisation, and as a result of that organisation's activities, and are calculated at the point of emission release.

[193] Scope 2 emissions (energy-related indirect emissions) occur outside the boundary of an organisation from the generation of electricity consumed by the organisation.

[194] DHAC (2023) *National Health and Climate Strategy*.

making. This is consistent with stakeholder feedback in the *National Health and Climate Strategy* stakeholder consultation.[195]

What we heard:

> 'Healthcare in Australia has a very large carbon and waste footprint ... We need change at pace and scale if we are to meet national targets for containing carbon emissions. This is an essential development described in this section.'
>
> **Consultation 2 submission: Professor Lynne Madden**

> 'Given the particularly harmful impact that the effects of climate change have on the health of people with asthma and the significant contribution of health technologies to climate change, appropriately resourced, planned and co-designed environmental impact reporting could be very positive on consumers.'
>
> **Consultation 2 submission: Asthma Australia**

Benefits of reporting greenhouse gas emissions associated with health technology products

Measuring and publicly reporting the greenhouse gas emissions associated with health technology products has two key benefits.

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 a range of competing products 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 4. Carbon Footprint of Common Inhalers Used for Asthma Management').[196]

The second benefit is that this information can be used to inform HTA 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

[195] DHAC (2023) *National Health and Climate Strategy - Consultation*, DHAC Consultation Hub.

[196] Bell K, Kazda L and Parker G (2023) 'Asthma in Adults', *The New England Journal of Medicine*, 389(22), doi: 10.1056/NEJMc2312345.

when such a decision might not otherwise have been warranted. A paper by McAlister et al.[197] 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.

*Table 4: Carbon footprint of common inhalers used to manage asthma**

Inhaler type	Clinical use	Drug class	Propellant	Example	Kilogram of CO_2e per inhaler
Pressurised MDI	Reliever	Large-volume SABA	HFA134a	Ventolin Evohaler (salbutamol)	25,260
Pressurised MDI	Reliever	Small-volume SABA	HFA134a	Salamol (salbutamol)	9,870
Dry-powder inhaler	Reliever	SABA	None	Bricanyl (terbutaline)	1
Pressurised MDI	Preventer	ICS–LABA	HFA227ea	Flutiform (fluticasone–salmeterol)	36,500
Pressurised MDI	Preventer	ICS–LABA	HFA134a	Fostair (fluticasone–salmeterol)	19,650
Dry-powder inhaler	Preventer	ICS–LABA	None	Advair Diskus (fluticasone–salmeterol)	1

** Data is based on the study by Wilkinson et al. CO2e denotes carbon-dioxide equivalent (a measure of global-warming potential), HFA134a hydrofluoroalkane 1,1,1,2-tetrafluoroethane, HFA227ea hydrofluoroalkane 1,1,1,2,3,3,3-heptafluoropropane, ICS inhaled glucocorticoid, LABA, long-acting β2-agonist, MDI metered-dose inhaler, and SABA short-acting β2-agonist.*

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 calculating the carbon footprint of health technology products are needed so that manufacturers can undertake a lifecycle assessment for each product just once, using an agreed methodology that is recognised by HTA bodies around the world. Existing standards for lifecycle assessment studies are not specific to health care (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 calculating the carbon footprints of pharmaceuticals. It has committed to developing an approach for considering environmental impacts as part of approval decisions by 2023–2024.[198] The Canadian Agency for Drugs & and Technologies in Health (CADTH) has committed to adapting methodologies and

[197] McAlister S, Morton RL and Barratt A (2022) 'Incorporating carbon into health technology assessments', *The Lancet Planetary Health* 6(12), pp. e993-e999, doi: https://doi.org/10.1016/S2542-5196(22)00258-3.

[198] National Institute for Health and Care Excellence UK (2021) *NICE Strategy 2021 to 2026 [PDF 1,970KB]*, NICE.

analysis to assess the environmental footprint of technologies as part of its 2022–2025 strategic plan.[199]

Australia has an opportunity, including through the international HTA collaboration, to shape the international regulatory environment by contributing to these efforts. In doing so, it ensures that environmental impacts are incorporated into HTA processes in an efficient and burden-minimising way. Failure to seize this opportunity creates a risk that Australia will have international standards imposed without being involved in their creation.

Conclusion

Australia should actively involve itself in efforts to establish new international standards for assessing the environmental impacts of health technology products. It should also consider requiring manufacturers to measure and publicly report emissions of products. This information should be incorporated into approval and reimbursement decisions, aligning with international best practice and developments in comparable countries.

Objectives of recommendations

The Review sets out new arrangements that recognise the substantial research and consultation conducted during the development of the *National Health and Climate Strategy*[200] and align with its four core objectives:

- **Health system resilience:** build a climate-resilient health system and enhance its capacity to protect health and wellbeing from the impacts of climate change
- **Health system decarbonisation:** build a sustainable, high-quality, net zero health system
- **International collaboration:** collaborate internationally to build sustainable, climate-resilient health systems and communities
- **Health in all policies:** support healthy, climate-resilient and sustainable communities through whole-of-government action that recognises the relationship between health and climate outcomes.

Recommendations

Recommendation 43. Environmental impact reporting

The Review recommends that the Australian Government, in line with the *National Health and Climate Strategy*, investigate the following options in consultation with industry and other stakeholders:

[199] Canadian Agency for Drugs & Technologies in Health (2022) *Ahead of the Curve: Shaping Future-Ready Health Systems 2022-2025 Strategic Plan*.

[200] DHAC (2023) *National Health and Climate Strategy*.

a. reporting of environmental impacts, starting with embodied greenhouse gas emissions, during the assessment of cost-effectiveness by Australian HTA bodies
b. potential to use this data in approval and reimbursement decisions
c. potential for public reporting of this data, to inform clinical decision-making
d. the development of guidance documents and examples to facilitate environmental impacts reporting
e. alignment with international best practice in comparable jurisdictions
f. the role of international standards for calculating the carbon footprint of health technology products.

Chapter 9: Supporting architecture for HTAs

Introduction

HTAs in Australia have traditionally been reactive; individual submissions are put forward (at the time of a sponsor's choosing) for marketing authorisation and/or public subsidy. This approach was considered an effective and appropriate way of making recommendations to the Government for the introduction of individual health technologies into health care in an era of slower technological development.

However, new health technologies are now being developed and introduced into clinical practice at an increasing pace and cost. And overall patient and clinician expectations for access to new health technologies (including for precision therapies that address clinical indications that previously had no treatments) are ever increasing. As a result, the HTA system is coming under pressure from time, resourcing and complexity perspectives, due to the need to:

- evaluate an increasing volume of health technologies at speed
- rely on smaller and/or less traditional evidence bases to support and make recommendations and decisions
- consider and implement recommendations arising from HTA processes in the context of an evolving and limited resource healthcare environment.

These pressures mean that for the HTA programs to support the objectives of universal health care as set out in the key pillars of the NMP,[201] a more proactive and contemporary approach to HTA processes (as set out in the recommendations from previous chapters) is required. However, for modern HTA arrangements to be efficient and effective in supporting patient access to health technologies, proper design and resourcing of the surrounding program, policy and stakeholder engagement and communications architecture that supports the HTA system is essential.

This chapter covers the Review's analysis, findings and recommendations on how a more proactive examination of, and adjustment to, a range of HTA-adjacent arrangements can support the responsiveness, agility and quality of the HTA process itself. These include:

- formalising approaches to proactively identify health technologies for areas of HUCN and elicit HTA submissions (Chapter 9.1)

[201] DHAC (2022) *National Medicines Policy*.

- improving the way the Australian health system identifies, monitors and plans for new health technologies in development via horizon scanning (Chapter 9.2)
- embedding arrangements to support regular HTA processes, methods and policy review so these keep pace with changes in HTA and health technology development (Chapter 9.3)
- integrating processes that support the development and application of HTA capacity and capability to meet increased demands (Chapter 9.4).

Chapter 9.1: Proactively addressing areas of unmet clinical need

Introduction and context

HTA processes in Australia depend on sponsors developing and lodging submissions to regulatory and/or reimbursement agencies. This means the HTA agenda is primarily set by the sponsors. This in turn can result in areas of unmet need.

Stakeholders have historically focused on addressing the need for improved access to health technologies for some Australian patients in under-represented or under-discussed clinical areas by:

- gathering direct inputs into the HTA process itself (such as consumer and/or clinician submissions during public consultation)
- making specific ad hoc representations to HTA advisory committees, government ministers or Commonwealth officers to support consideration of facilitated 'workarounds' and/or direct sponsorship of individual health technologies for subsidy and supply. This has been most commonly observed in areas relating to First Nations health and paediatric health technologies.

This is a shortcoming of the current processes and results in inequities. To address this, the Review considered whether policies, programs and processes outside, but adjacent to and/or supportive of the HTA evaluation process, could be created or improved so they:

- improve how health equity is considered and practically addressed by introducing high added therapeutic value technologies in areas with HUCN
- improve the quality of HTA and health policy advice for specific health technologies and health equity priority areas
- support improved and more expansive stakeholder engagement and co-design arrangements, including health literacy efforts.

Why this matters

A more forward-looking approach to identifying areas of HUCN improves the Australian Government's ability to engage earlier with stakeholders to identify and support the provision of subsidised health technologies for under-served populations, and address objectives of improving health equity.

Findings

In stakeholder consultations, the Review received consistent feedback that Australia should adopt a more proactive approach to identifying therapies that address HUCN, in a manner that ensures proper stakeholder participation in the overall process.

Case examples of comparable approaches include programs in Canada (via CADTH) and the UK (via the UK National Health Service, the National Institute for Health and Care Research Innovation Observatory (NIHRIO) and NICE). These use a mix of horizon scanning, PICO scoping and other proactive stakeholder engagement activities to support engagement on matters of health equity and implications that may arise from the possible introduction of new health technologies into routine health care.

What we heard:

'Develop a national strategy that is informed by disease burden and unmet need, and is integrated with strategies for funding innovative medicines ... Importantly, this should not neglect rare diseases and unmet need.'

Consultation 1 submission: Biointelect

'There is a lack of horizon scanning and pre-emptive planning of new and emerging technologies. Submissions are reactive, from the local sponsor, and are not able to be driven proactively by patient or healthcare needs.'

Consultation 1 submission: South Australian Department for Health and Wellbeing

The Review consulted on a suite of ideas[202] supporting the development of a priority list of HUCN areas that would be used to inform horizon scanning, preliminary assessment and prioritisation of dialogue with stakeholders. This would inform explicit

[202] DHAC (2023) *HTA Review Consultation 2 Options Paper*, Health Technology Assessment Policy and Methods Review.

invitations to sponsors to bring health technologies addressing HUCN forward for regulatory and reimbursement consideration.

While stakeholder support was strong for the proposed ideas, stakeholders raised important observations and criticisms that informed further Review deliberation:

- Care would be needed when developing and using the priority list, to ensure no loss of health equity (due to displacement and/or deprioritisation of treatments for areas of non-HUCN).[203]
- Developing and maintaining the priority list would need to be done in partnership with all key stakeholders.[204]
- Clarity and flexibility about the mechanisms and criteria supporting proactive assessment and sponsor invitations would be required, to give stakeholders sufficient opportunities to consider all necessary factors and implications associated with sponsoring an HTA submission in a HUCN area.[205]

Conclusion

Australia's health system will not be able to fully and effectively meet the NMP's[206] objectives of ensuring that all citizens have equitable access to safe, effective and high-quality medicines, culturally appropriate medicines-related services and medicines-related information, as long as gaps remain in the way the health system:

- proactively identifies areas of unmet clinical needs
- examines and discusses with stakeholders the need for, and supporting activities necessary to encourage, the evaluation and provisioning of potential therapies that may address those clinical needs.

The process of designing and developing criteria for identifying therapeutic areas of HUCN is important to support:

- improved (and earlier) stakeholder engagement and participation, including information sharing between stakeholder parties
- improvements in stakeholder literacy in relation to health technologies, clinical indications and health equity–related policies and strategies (including, for instance, the National Antimicrobial Resistance Strategy[207] and the National Strategic Action Plan for Rare Diseases[208])
- informed planning and design of HTA submissions and their introduction into the Australian healthcare context (including informing a range of HTA process-

[203] See AbbVie's submission to Consultation 2 for a representative view of this observation.

[204] See *Consensus Letter from 51 consumer organisations* as part of Consultation 2 for a representative view of this observation.

[205] See Roche's submission to Consultation 2 for a representative view of this observation.

[206] DHAC (2022) *National Medicines Policy*.

[207] Australian Government (2020) *Australia's National Antimicrobial Resistance Strategy – 2020 and Beyond*.

[208] DHAC (2020) *National Strategic Action Plan for Rare Diseases*.

related supporting activities such as pre-submission dialogue and early PICO scoping), especially for health areas that may not have historically attracted the same attention as more high-profile clinical areas like oncology or cardiovascular diseases (e.g. paediatric indication extensions)
- public signalling in areas with strong interest in improving knowledge about, and availability of, specific health technologies (e.g. products to address antimicrobial resistance, paediatric indications and First Nations health needs), as part of improving overall subsidised access to treatments in areas of HUCN.

While it is important to identify therapeutic areas of HUCN, it is essential to translate outputs into specific arrangements and activities that support the introduction of health technologies into Australia. In particular, the recommendations associated with proactive invitations for HTA submissions are designed to give practical effect to the upstream prioritisation, research and consultation activities in a way that improves health equity and helps address the treatment needs of patients in areas of HUCN.

Objectives of recommendations

The Review's recommendations set out new arrangements that focus on identifying, screening and inviting the submission of health technologies for public subsidy that address HUCN and health inequities.

Recommendations

Recommendation 44. Identifying therapeutic areas of high unmet clinical need

The Review recommends that the Australian Government:

a. develop criteria for ongoing identification of therapeutic areas of high unmet clinical need (HUCN) in partnership with clinicians, industry, patients and patient and/or community organisations to:
    i. inform further stakeholder dialogue, horizon scanning and related proactive pre-HTA activities that improve health literacy and health equity
    ii. provide a mechanism to publicly signal the need for, and gauge interest in, making available and subsidising certain health technologies that address specific therapeutic needs in the Australian healthcare context (such as First Nations and paediatric health), but are not available due to limited commercial and operational interest from sponsors
b. in developing and consulting on the criteria, have regard to:
    i. priorities developed through other government activities (such as, but not limited to, outreach activities supporting the Medical Research Future Fund (MRFF))

ii. antimicrobial resistance surveillance information to identify emerging resistance to available treatments, and surveillance of vaccine preventable diseases.

c. develop agreed processes that support regular review of and updates to criteria and therapeutic areas
d. support a subset of the criteria being developed in partnership with Aboriginal and Torres Strait Islander Community Controlled Health Services (ACCHSs) to identify priority areas of HUCN for First Nations people, in line with the priority reforms under the National Agreement on Closing the Gap between all governments and the Coalition of Peaks.

Recommendation 45. Identifying therapies to address therapeutic areas of high unmet clinical need

The Review recommends that the Australian Government develop a process consistent with the principles of horizon scanning (see Chapter 9.2) for identifying therapies with the potential to be high added therapeutic value for therapeutic areas of high unmet clinical need (HUCN), including:

a. new therapies that may not be available in Australia
b. existing therapies with initial evidence that they could be repurposed for new indications
c. existing therapies with initial evidence that changes to an existing restriction and/or authority may address HUCN and/or significant health inequity
d. includes in this process a mechanism for partnering with Aboriginal Community Controlled Health Services (ACCHSs) to ensure First Nations population health outcomes and health equity are appropriately reflected.

Recommendation 46. Proactive pre-HTA processes supporting the introduction of identified health technologies for high unmet clinical need

The Review recommends that the Australian Government:

a. establish processes that facilitate proactive dialogue between stakeholders to support the timely development and lodgement of HTA submissions for health technologies that meet the relevant eligibility criteria. Health technologies discussed would be nominated in consultation with clinician and consumer stakeholders after being identified through the horizon scanning process and the resulting outputs of the process (see 'Recommendation 44. Identifying therapeutic areas of high unmet clinical need')
b. consult on, develop and apply (as appropriate) incentives that will support the development and lodgement of HTA submissions for these therapies, including (but not limited to):

i. facilitated Population, Intervention, Comparator, and Outcomes (PICO) scoping and development
ii. fee waivers
iii. case management support
iv. prioritised pathway access (through proposed new proportionate HTA pathways)
v. potential for access to bridging funding programs (subject to HTA advisory committee recommendation)
vi. data exclusivity arrangements (where applicable).

c. establish process protocols for sponsors with identified health technologies to:
   i. notify their intention to prepare submissions to the Pharmaceutical Benefits Advisory Committee (PBAC) (and application to the Therapeutic Goods Administration (TGA), if applicable) within a predefined period after official invitation by the Australian Government (to be determined in consultation with stakeholders)
   ii. submit and publish project plans detailing the timing of key milestones supporting the preparation and lodgement of HTA submissions for the health technology(ies).
d. in cases where market incentives do not attract submissions for therapies for high unmet clinical need (HUCN), consult with stakeholders on potential mechanisms to support registration and access.

Chapter 9.2: Horizon Scanning

Introduction and context

Horizon scanning in the healthcare context broadly describes a process that helps 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.

Australia does not perform any formal horizon scanning activities in health care at the national level, and does not fund horizon scanning arrangements. Some horizon scanning activities were historically sponsored through the Health Policy Advisory Committee on Technology (HealthPACT) and subsequent advisory committee arrangements. These arrangements reported to the Australian Health Ministers' Advisory Council before its dissolution in 2020.

During The New Frontier inquiry, stakeholders expressed varying degrees of concern that Australia no longer has any formalised horizon scanning arrangements at the national level. This contrasts with comparable health systems that either have localised nationally sponsored horizon scanning programs (e.g. CADTH and NIHRIO) and/or participate in cross-border horizon scanning collaborations (e.g. the International Horizon Scanning Initiative).[209]

It is important to examine the prior experiences and current use of horizon scanning locally and in international contexts, to ensure Australia's HTA policy, processes and methods are well adapted, and capable of efficiently and effectively assessing new and emerging health technologies.

Why this matters

Systematically collecting, analysing and sharing information on health technologies in the research pipeline and entering clinical practice will allow stakeholders to be better informed about the benefits, risks and operational implications of new health technologies. It will also prompt earlier conversations about health systems planning to support the introduction of new health technologies.

[209] See comment from Chair of the PBAC and from Roche on p305 for representative views of these observations, Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

Findings

Stakeholder reflections on the purpose of horizon scanning can be grouped broadly into one or more areas:

- identifying future products and technologies in therapeutic areas
- gathering patient insights about health technologies of interest
- identifying gaps in knowledge and data relevant to a given health technology
- informing assessment pathways (and necessary flexibility and/or modifications) relevant to future health products and technologies (especially for cell and gene therapies)
- informing decisions about health system resourcing and preparedness to support the introduction or adoption of new health technologies.[210]

Similarly, the stated purposes of horizon scanning arrangements conducted by comparable overseas HTA agencies were broadly grouped into the following areas:[211]

- identifying 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 of:

- identifying topics
- filtering topics
- selecting (prioritising) topics for further attention.

Stakeholder responses to proposed options for reintroducing horizon scanning and increasing horizon scanning capacity and capability in Australia were mostly positive about intent and purpose (i.e. supporting better visibility of health technology development for more proactive HTA evaluation and health system planning, and to improve health literacy about products in development). These stakeholders also noted the importance and necessity of proper resourcing of a new horizon scanning program to achieve intended outcomes, given the resource-intensity of a well-designed

[210] See Cancer Health Services Research Unit, University of Melbourne submission to Consultation 1 for a representative view of this observation.

[211] Adelaide Health Technology Assessment (2023) *Horizon Scanning and Early Assessment*, Health Technology Assessment Policy and Methods Review within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

process.[212] The Review did not receive specific feedback about the quantitative resourcing requirements of a horizon scanning program. However, examination of public financial statements from organisations that conduct horizon scanning–type functions (such as NIHRIO and the US Patient-Centered Outcomes Research Institute) suggest ongoing investment of around A$3 million or more annually tends to be the minimum resourcing level,[213,214] noting that resourcing correlates to the program's defined functional scope, audience and purpose.

A number of stakeholders mentioned including specific healthcare areas (such as First Nations health care, paediatrics and the intersection between genetic testing and use of health technologies, including combination therapies) in the scope of horizon scanning arrangements as activities that would support overall improvements to health equity.[215] Stakeholders specifically mentioned these healthcare areas as they have historically received less attention, and/or are areas of increasing innovation and change that may require more active monitoring and engagement with stakeholders.

What we heard:

'There was some really specific positives around the focus on First Nations, proactive horizon scanning, consideration of equity and an emphasis on the inclusion of patient groups in the HTA process.'

'The reforms around the role of genetics is very important. The system needs to be futureproof so that efficiencies can be made in the system. We ultimately don't want people dying from lack of access to medicines/tests that have been approved in other indications. This is where horizon scanning would help as technology could be listed for a number of indications at the same time.'

Quotations attributed to stakeholders present at Consultation 2 webinars and in-person sessions

Conclusion

Stakeholder feedback from patients, industry, service delivery and healthcare payers consistently emphasised the importance of horizon scanning. In particular, feedback highlighted its role in supporting and complementing the efficient evaluation, resource

[212] See Medicines Australia and Breast Cancer Network Australia submissions to Consultation 2 for representative views of these observations.

[213] National Institute for Health and Care Research UK (2024) *NIHR Annual Report 2022/23*.

[214] Patient Centred Outcomes Research Institute (2023) *2023 Financial Report and Audit*.

[215] See Neurological Alliance Australia and Asthma Australia submissions to Consultation 2 for representative views of this general observation.

allocation, stakeholder engagement and policy planning activities associated with using health technologies in the Australian health system.

Drawing on this feedback and international examples discussed in *Horizon Scanning and Early Assessment*,[216] horizon scanning arrangements have a role as part of HTA-adjacent supporting activities and functions. Key baseline characteristics for such a program need to be identified and agreed with stakeholders.

The following observations about horizon scanning should assist the Australian Government as it engages in implementation activities with stakeholders:

- The scope, purposes and utility of horizon scanning will need to be clearly defined and planned with stakeholders (participants and recipients of outputs) to ensure success in the Australian healthcare context. These include careful consideration of whether specific program streams are necessary to support improved health equity, from the perspectives of stakeholder engagement and participation, and of practical access to health technologies, especially for areas of HUCN. The Review has signalled examples where prioritising horizon scanning activities may be beneficial.
- Horizon scanning can be highly resource-intensive, in terms of financial costs, time and effort (for in-kind engagement and inputs by partner stakeholders such as patients and industry sponsors). All participating and benefiting stakeholders will need to be mindful of these resourcing costs when advising and working with the Australian Government on implementation.
- Multiple streams of horizon scanning activities may need to be supported to ensure:
    - appropriate consideration of the different health technology issues and varying target audiences and stakeholders (such as, but not limited to, patients and their advocacy organisations, industry sponsors, healthcare payers, and academic and/or research bodies)
    - proper establishment of clear metrics to determine the applicability and success of these streams of work in improving engagement and advice about awareness, evaluation and access to health technologies in the medium term.
- Building local capacity and capability may take some time. In the short term, to increase engagement and partnership with other jurisdictions and international HTA agencies, it may be necessary to emphasise collaborative work-share arrangements and/or sourcing of external (i.e. not in-house Commonwealth) expertise.

[216] Adelaide Health Technology Assessment (2023) *Horizon Scanning and Early Assessment*, Health Technology Assessment Policy and Methods Review within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

Objectives of recommendations

The Review's recommendations set out new arrangements that focus on identifying health technologies and emerging health systems issues in a structured form. This will support efficient evaluation, resource allocation, stakeholder engagement and policy planning activities associated with using health technologies in the Australian health system.

Recommendations

Recommendation 47. Horizon scanning

The Review recommends that the Australian Government:

a. establish an Australian horizon scanning function that supports the broad principles of:
    i. improving the quality of HTAs, health policy and stakeholder engagement arrangements when considering the implications of new and emerging health technologies
    ii. improving stakeholder awareness and engagement about technologies that may address important healthcare areas (e.g. high unmet clinical need (HUCN), national healthcare priorities and health equity considerations)
    iii. supporting advice that helps healthcare payers with forward planning and setting priorities.
b. work with key stakeholders to ensure that the scope, audience, purpose, governance and outcomes of horizon scanning are appropriately designed so that information can be used to support evidence-based recommendations and advice that support improvements to health technology access and availability for Australian citizens
c. prioritise horizon scanning activities in areas where early attention is most likely to identify major health advances that address health inequities and HUCN and/or have significant health system implications, including (but not limited to):
    i. advanced therapies and health technologies that require collaboration between multiple healthcare payers and providers (including, but not limited to, states and territories)
    ii. health technologies that may support improvements in health equity (including, but not limited to, First Nations health and areas of HUCN) and national health priority areas.
d. provide adequate resourcing to support effective and efficient ongoing operation of the program after open engagement with stakeholders about costs and related contributory implications

e. establish appropriate governance arrangements after consulting stakeholders, and put in place arrangements to:
    i. review the horizon scanning function periodically
    ii. support necessary dialogue with stakeholders to adjust the function's operation over time to ensure it continues to provide efficient and effective outcomes that meet the agreed purpose(s) and scope(s).

Chapter 9.3: Mechanisms for continuous review and improvement

Introduction and context

Australia's HTA processes and methods have continuously evolved since the introduction of HTAs in Australia in 1993. This includes updates to HTA guidance, policy and processes.

From the perspective of reviewing and improving processes, commitments to streamlining medicines listing procedures and changes to cost-recovery arrangements are set out in the two most recent strategic agreements with Medicines Australia.[217] These include publishing and reporting against agreed metrics and key performance indicators (KPIs).

However, the approach to formally reviewing and improving HTA arrangements (at technical, process and policy levels) can be irregular. It is also often driven by specific external circumstances, rather than as part of a continuous process or program of work. For example:

- While HTA advisory committee guidelines have been changed a number of times since they were established, there is no formal or systematic schedule for reviewing them. Major updates take place at 6- to 10-year intervals.
- Before this Review, the last HTA review was undertaken by the then Department of Health and Ageing in 2009. Its focus included streamlining the arrangements for the then Prostheses List (now the Prescribed List of Medical Devices and Human Tissue Products), improving the rigour and efficiency of the MSAC, and improving the transparency and post-market surveillance of health technologies.
- Post-market review (PMR) findings can also on occasion prompt changes to policy relating to medicines evaluation or supply.

[217] DHAC (2022) *Strategic Agreement between the Commonwealth of Australia and Medicines Australia 2022-2027*, PBS.

In response to these observations, the Review investigated whether approaches to support continuously reviewing and improving HTA-related policies, guidelines and processes could be created or more formally embedded into departmental arrangements to:

- limit the future need for major reviews (such as this Review) to be the key driver for improving HTA arrangements
- ensure Australia's HTA policy, methods and processes continue to keep pace with rapid advances in health technology
- ensure timely updates to HTA guidelines arising from reviews of HTA policy, methods and processes to provide necessary clarity and transparency for key stakeholders and to support confidence in the outputs of the HTA arrangements.

Why this matters

Without regular review of HTA policy and methods, the Australian HTA arrangements may not be positioned to provide the best HTA advice to the Government to inform its decision-making about whether to subsidise a new health technology for patients, and the conditions of subsidy.

Findings

The Review heard initially from stakeholders that the rapid development of new health technologies, evolving clinical place of health technologies and need for new methods to evaluate newer health technologies with less traditional evidence bases means that it is necessary to review and update HTA guidelines more frequently.

In response to these observations, the Review tested with stakeholders the merits of recommending a program of continuous review and improvement for HTA policy and methods. This included ideas for a rolling set of review topics and process guidance to support regular updates to guidance documents (such as the HTA advisory committee guidelines) and HTA-related policy or programs of work (e.g. approaches to post-HTA data collection and evaluation) as health technologies and methodologies evolve.

Most individuals and organisations that responded to supplementary consultations supported a program of rolling reviews of Australia's HTA policy and methods. Many submissions mentioned continuous review and improvement as pivotal to the long-term success of the HTA system to ensure it can constantly meet the needs of the rapidly evolving HTA and health technology landscape.[218]

[218] See AbbVie's submission to Consultation 2 for a representative view of this general observation.

A subset of stakeholders suggested making additional amendments to the recommendations to emphasise:

- the importance of consulting with, and seeking input from, stakeholders throughout any review process[219]
- the need for KPIs and other specific measurements to allow assessment of whether changes to the system arising from the Review's recommendations achieved their intended outcomes.[220]

Conclusion

It is essential to have a program to continuously review HTA policy and methods (including associated guidelines and supporting documentation) to keep pace with changing product development, and the HTA and research landscapes. This program should include scheduled examinations of individual HTA-related topics that affect the operations of the HTA system. It should also be sufficiently responsive to be able to address new developments in HTA methods or health technology that may require changes to documentation and/or policy outside scheduled review time frames.

The Review acknowledges the stakeholder feedback regarding the importance of KPIs and specific measurements that allow comparative analysis of how changes in HTA policy, methods and guidance affect the performance of the HTA program and the desired overall outcomes in the central pillars of the NMP.[221] Specifying the details of these KPIs (other than the timeliness targets for products demonstrating superiority outlined in 'Recommendation 15. Jointly owned performance targets') is beyond the scope of the Review. But it is clear that it will be necessary to consult with external and internal stakeholders to ensure the right details are measured, collected and reported, to inform identification of areas where further review and improvements are required, and the sequencing of the review program itself.

Objectives of recommendations

The Review's recommendations emphasise the need for a clear program of work that supports regularly updating and reviewing HTA policy and methods in light of new developments. This will keep Australia's HTA process, methods and policy up to date and can support rigorous and high-quality evaluation of health technologies.

[219] See Novartis Australia's submission to Consultation 2 for a representative view of this general observation.

[220] See Roche's submission to Consultation 2 for a representative view of this general observation.

[221] DHAC (2022) *National Medicines Policy*.

Recommendations

Recommendation 48. Mechanisms for continuous review and improvement

The Review recommends that the Australian Government design and establish (in consultation with stakeholders) a program that supports the continuous review and updating of HTA policy and methods in support of the core pillars of the NMP. This program would include:

a. a selection of review topics informed by:
    i. consultation with internal and external stakeholders on areas where systematic concerns have been identified
    ii. contemporary research into international and interjurisdictional best practice
    iii. findings from key performance indicators measurement and reporting arrangements.
b. a transparent schedule of topics for review and review consultation activities, and designated time frames to complete reviews
c. opportunities for all stakeholders to provide input to reviews
d. reporting of review outcomes and (where necessary) recommendations that (if implemented) would improve the operation of the HTA program.

Chapter 9.4: Capacity and capability of the HTA system and supporting architecture

Introduction and context

Several emerging areas are challenging the existing HTA workforce. These include:

- increasing numbers and types of health technologies being brought forward for HTA evaluation each year
- greater challenges in assessing health technologies, due to increased complexity and changes to the size and quality of the evidence base over time
- greater need for developing and re-evaluating evidence after health technologies have been funded
- changes in evaluation approaches that may require upskilling of evaluators to keep pace with best practice methods and processes
- increasing interest among HTA bodies internationally in opportunities for work-sharing and parallel HTA evaluation arrangements as part of process efficiencies.

The New Frontier inquiry 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.'[222]

Resourcing of the Australian HTA workforce is important to maintain its capacity and capability to evaluate evidence provided in submissions. However, it is also essential that the HTA evaluation process itself is properly supported by the range of activities and functions that are essential to give practical effect to the HTA advisory committee's recommendations and enable subsidised access to health technologies. These other activities and functions include price negotiation arrangements, listings processes and post-subsidy review functions (including further stakeholder engagement on matters of access and disinvestment). These may in turn inform future HTA advisory committee deliberations.

In response to these observations, the Review agreed that it was important to examine resourcing-related implications for the HTA evaluation process and the surrounding HTA-supporting work programs. This includes, but is not limited to, price negotiation, stakeholder engagement and communications, listings processes and pre-HTA evaluation activities discussed in this Review, as well as post-HTA reassessment functions.

Why this matters

The availability, quality and proper alignment of workforce capacity and capability for:

- the HTA evaluation process
- the supporting pre-HTA activities that improve the quality and efficiency of the HTA process
- the activities that support translation of HTA recommendations into subsidised health technology access

all influence the effectiveness and efficiency of delivering the NMP's objectives for timely access to health technologies.

(That is, insufficient resourcing and/or misalignment of resourcing can slow down key parts of the process that support access to medicines for patients.)

[222] Recommendation 5, Australia Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) *The New Frontier inquiry*.

Findings

General observations

Australia's HTA workforce is highly skilled and capable of addressing evaluation needs in support of the HTA advisory committees. However, much of this capacity and capability is based on the extensive on-the-job training experiences of personnel, built up since economic evaluation for the Australian HTA context was introduced in the mid-1990s.

As noted in a number of submissions to the Review, and in discussions with HTA evaluator groups, developing the necessary experience and basic competence in HTA evaluation requires a mix of health economics study and several years' experience with handling HTA submissions. Additional training and research activities necessary to improve HTA methodologies and their application (including consistent approaches to evaluation) require additional time and resources, which are not readily available or provided in Australia.[223]

In response to these observations, the Review tested stakeholder appetite for improving the capacity and capabilities of the HTA workforce via a specially designed and certified HTA evaluator training and work experience program (i.e. sponsored internships), drawing on comparable mixed education and work experience programs used in other workforce sectors. Under this program, identified individuals would be given additional targeted training, coursework and exposure to the range of HTA-related activities and different stakeholder perspectives (academic, government and industry) that underpin the HTA system in Australia.

There was limited opposition to the concept of sponsored internships as a way to upskill and broaden the HTA personnel base. But concerns were raised about appropriate remuneration for internships, especially for mid-career students who may already be in full-time employment.[224] Another concern was whether internship programs could be broadened to industry settings to provide wider experience and perspective to the HTA process.

Some of the other proposed reforms (including streamlining HTA evaluations and horizon scanning) are expected to require significant additional workforce capacity and capability. So, the Review was heartened by stakeholder feedback supporting a review and overhaul of resourcing for the HTA arrangements to meet the needs of proposed new functions and activities put forward during consultations.

[223] See Research Australia's submission to Consultation 1 for representative views of these observations.

[224] See Asthma Australia's submission to consultation 2 for a representative view of this observation.

The possibilities arising from international work sharing and collaboration

In briefings and discussions with international HTA entities and with the TGA, the Review noted evidence of an increasing volume and scope of joint HTA evaluation by agencies in different countries (i.e. international work sharing) for reasons of efficiency and consistency.

If well organised and constructed, Australian participation in these international work-sharing arrangements would likely increase the capacity and capability of the local HTA workforce.

Feedback from the TGA Medicines Regulation Division noted that the opportunities to use evaluation reports from comparable overseas regulators,[225] and to participate in parallel work-sharing evaluation arrangements with other comparable regulatory agencies (via the Access Consortium[226] and Project Orbis[227]), would improve the capability of the evaluation group. They would do this by:

- providing opportunities to draw on the experiences of a broader HTA evaluator cohort and test evaluation logic and approaches to analysing evidence
- spreading the effort of evidence analysis among different work groups while allowing access to important quality check and peer review resources to improve evidence analysis and final recommendations.

Similar examples of work-sharing arrangements identified during the Review, but in support of reimbursement-related HTA evaluation, include the Nordic FINOSE collaboration, BeneLuxa Initiative and the forthcoming EU Health Technology Assessment Regulation legislation supporting joint clinical assessments.[228] These all have improvements to workforce capability and capacity as part of their respective remits and objectives (via sharing of evaluation workload).

Stakeholders generally supported international partnerships and work sharing among HTA agencies to improve HTA-related capacity and capability. But industry stakeholders were generally opposed to international partnerships that would address matters relating to harmonised pricing and joint procurement.[229] Additionally, some stakeholders emphasised the importance of clarifying the scope of work and

[225] DHAC (2019) *Comparable overseas regulators (CORs) for prescription medicines*, Therapeutic Goods Administration (TGA).

[226] DHAC (2023) *Australia-Canada-Singapore-Switzerland-United Kingdom (Access) Consortium*, TGA.

[227] DHAC (2023) *Project Orbis*, TGA.

[228] Additional details on these arrangements can be found in Adelaide Health Technology Assessment (2023), *International Health Technology Market Approval, Funding and Assessment Pathways*, Health Technology Assessment Policy and Methods Review within Adelaide Health Technology Assessment (2024) *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*, Health Technology Assessment Policy and Methods Review.

[229] See BMS Australia and Novartis Australia submissions to Consultation 2 for representative views of this observation.

addressing unintended implications that may arise from possible sharing of commercially sensitive information between HTA agencies.[230]

Conclusion

HTA evaluator workforce

The pressures on HTA capacity and capability in Australia are due to the increasing HTA workload (in volume and complexity), and the lack of a clear pipeline of prospective evaluators to ensure appropriate levels of continuity and succession. Addressing these pressures will require long-term support and contributions from all key stakeholders, as developing the capability and expanding the capacity of a highly skilled HTA workforce will take years, rather than weeks or months.

It will require a combination of:

- creating opportunities for on-the-job training and skills development for new and existing evaluators (external and internal to the Department) via greater exposure to a diverse range of HTA submissions, secondments with other work programs or stakeholder groups, and collaborations with international evaluators
- establishing and embedding a clearly defined pathway for prospective and existing evaluators to learn and apply coursework and new learnings in a 'live' public program environment as part of developing understanding about the HTA process, broader HTA policy and new HTA methods that may be relevant to evaluating new health technologies
- drawing on existing local HTA capacity and capability differently, including through possible pooling of HTA capacity within government departments to do HTA evaluations and/or support discrete HTA-related activities such as PMRs.

Providing appropriate support structures (including funding and time) to allow evaluators (and prospective evaluators) to engage in continuous development and training will be essential to the long-term sustainability of the HTA workforce. This will need to be accounted for as part of HTA resource planning and allocation of submissions to evaluators in the short to medium term.

The Review also strongly supports efforts to improve the scope and volume of work-sharing opportunities with comparable international HTA agencies. This is a practical way to exchange information and ideas between workforces and improve their capability, as well as the quality of HTA outcomes overall.

Components of the workforce-related recommendations proposed in this report are complex and will require careful consideration, consultation and analysis before implementation. They may also (in the short term) require the same evaluator

[230] See Medicines Australia's submission to Consultation 2 for a representative view of this observation.

stakeholders conducting HTAs to set aside time and effort to engage effectively with the complexities of implementation. However, this may be a necessary temporary sacrifice to ensure that reforms supporting workforce development and capacity building are embedded properly to yield long-term benefits to the HTA system.

HTA-related and adjacent functions necessary to support access to health technologies

The Review received positive stakeholder feedback on a number of options that would require significant investment in the workforce in areas that are distinct from the evaluation workforce, but essential to an efficient and effective HTA system. They are also vital for the HTA system's role in improving access to health technologies for patients, at a cost individual and the community can afford.

The Review emphasises that its recommendations – ranging from those for enhanced stakeholder engagement and collection and use of evidence, to new functions such as horizon scanning and operating a bridging fund – cannot be delivered effectively within the current Departmental appropriations. Additionally, there must be sufficient opportunity to examine and consult on how best to efficiently and flexibly allocate resources to these activities (including options for pooling personnel with jurisdictional partners for specific matters). As the Australian Government considers the recommendations in this report, the Review strongly encourages careful consideration of the appropriate capacity and capability for supporting functions that are essential to support quality HTA submissions, and efficient evaluation and translation of HTA recommendations into practical health technology access for patients.

Objectives of recommendations

The Review's recommendations emphasise the importance of a having well-resourced and capable HTA program and architecture to support:

- efficient and effective evaluation of health technologies
- delivery of supporting functions and activities required to facilitate access to, and understanding about, health technologies, in line with the NMP pillars and principles.[231]

Recommendations

Recommendation 49. HTA evaluation workforce

The Review recommends that the Australian Government:

a. consult broadly to develop programs that enhance the competency and capability of the HTA workforce, including (but not limited to):

[231] DHAC (2022) *National Medicines Policy*.

i. sponsored internships between HTA evaluation groups, health departments and industry
ii. facilitated secondments between HTA evaluation groups, health departments and industry
iii. international secondments between HTA collaboration countries.

b. discuss with state and territory health departments, opportunities for developing an inter-government evaluation work group to improve capability development and use of HTA capacity as part of achieving nationally consistent HTAs
c. continue progress on inter-agency collaboration and design relating to common HTA evaluation methodology, as part of supporting testing and (prospective) formal introduction of HTA evaluation work-sharing pathways across participating jurisdictions
d. approve reforms to pilot work-sharing pathways for individual health technology submissions that are submitted across jurisdictions with comparable approaches to HTA evaluation. This would reveal the merits of collaborative evaluation for reimbursement-related activities that – if the experience is positive – could be embedded into the HTA framework. Available pathways should include at least one of the following options:
    i. **Work-sharing initiative pathway** – concurrent reimbursement submissions are lodged in multiple jurisdictions and work on dossier modules is split among participating agencies.
    ii. **Comparable overseas agency pathway** – finalised HTA evaluations from comparable agencies are provided for review (with redactions for localised pricing information, as strictly necessary).
    iii. **Joint expression of interest HTA pathway** – 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).
    iv. **Hybrid sequential lodgement pathway** – 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.
e. update its parallel scientific advice and 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 (locally or regionally), where a joint HTA evaluation is under consideration.

Recommendation 50. Supporting architecture resourcing

The Review recommends that the Australian Government give careful consideration to the quantity and alignment of resources (financial and personnel) required to

effectively implement any agreed recommendations arising from this Review. This includes:

a. appropriations for new activities
b. additional resourcing necessary to reform and/or strengthen existing functions that are essential to support the HTA process and translate HTA recommendations into health technology access for patients. These functions include, but are not limited to:
    i. health communications expertise
    ii. enhanced stakeholder engagement
    iii. commercial negotiation
    iv. HTA evaluation
    v. triaging and case management support.

Glossary

Words and phrases used in the Review are to be interpreted as follows (unless otherwise specified):

Term	Definition
2020–25 Addendum to the National Health Reform Agreement (NHRA)	An agreement between the Australian Government and all state and territory governments that commits to improving health outcomes for Australians, by providing better coordinated and joined-up care in the community and ensuring the future sustainability of Australia's health system. It is the key mechanism for the transparency, governance and financing of Australia's public hospital system. Through this agreement, the Australian Government contributes funds to the states and territories for public hospital services. This includes services delivered through emergency departments, hospitals and community health settings.
Australia's National Medicine Policy (NMP) 2022	A high-level framework focused on the availability and use of medicines and medicines-related services. The NMP relates to medicines research and development, manufacture, regulation, evaluation, supply, dispensing, storage and access. It promotes the quality use of medicines and medicines safety by focusing on the current and future health needs of people and the responsibilities of all partners to achieve the best health, social and economic outcomes for all Australians.
Cabinet	The Cabinet is the council of senior ministers empowered by the Australian Government to take binding decision on its behalf.
Cell therapy	The transfer of cells into a patient with the goal of treating a disease. The cells may be from the patient or from a donor. Cell therapies include gene modified cell therapy, which involves removing cells from a patient's body, to introduce a new gene or correct a faulty gene in vitro. The modified cells are then put back into the body.
Consumer (health)	A person who uses (or may use) a health service, or someone who provides support for a person using a health service. Consumers can be patients, carers, family members or other support people.
Consumer Consultative Committee	The Consumer Consultative Committee is a committee that provides strategic advice and support to the principal Commonwealth HTA advisory committees. It brings consumer views into HTA processes and relevant matters.
Consumer Evidence and Engagement Unit	A unit that was set up in 2019 to develop structured engagement projects with consumer and patient groups. The unit creates opportunities for consumers and patients to contribute to HTA

	decision-making processes. In addition, the unit is also the Secretariat for the Consumer Consultative Committee.
Conversations for Change	A series of consultations held by the Consumer Evidence and Engagement Unit. The consultations considered how to improve the way the Department of Health and Aged Care communicates and engages with, and includes, consumers and carers in HTAs.
Cost-effectiveness analysis	An economic evaluation that compares health technologies that have a common health outcome, in which costs are measured in monetary terms and the outcome is measured in natural units.
Cost-minimisation analysis	An economic evaluation that identifies the least costly health technology after the proposed health technology has been demonstrated to be no worse than its main comparator(s) in terms of effectiveness and toxicity.
Cost-utility analysis	An economic evaluation that compares health technologies in which costs are measured in monetary terms, and outcomes are measured in terms of extension of life and the utility value of that extension (such as quality-adjusted life years or healthy-year equivalents).
Effectiveness, clinical	The extent to which a health technology produces its intended outcome(s) in a defined population in uncontrolled or routine circumstances.
Enhanced Consumer Engagement Process	A commitment in the Strategic Agreement between the Commonwealth and Medicines Australia that the Commonwealth work with Medicines Australia, and consumer, clinician and other stakeholder groups, to co-design and agree upon an Enhanced Consumer Engagement Process to capture consumer voices in relation to applications to list new medicines on the Pharmaceutical Benefits Scheme. This co-designed process could reduce the likelihood of multiple reimbursement submissions by assisting the Pharmaceutical Benefits Advisory Committee and other independent HTA advisory bodies, at an early stage, to obtain an understanding of issues arising from new technologies, innovations and associated implications for consumers.
Equity	The principles of fairness informing decision-making. Also refer to the NMP description of 'equity'.
Evaluation (for HTA advisory committees)	Refers to the process undertaken to assess the clinical, economic, financial and use aspects of health technologies.
First-in-class technology	A first-in-class medicine or vaccine, and/or a medicine or vaccine for a new population. • A first-in-class medicine or vaccine represents a drug or vaccine with a unique mechanism of action that has not been considered by the PBAC.

	• A new population could include a disease or medical condition not previously considered by the PBAC. • A disease is intended to cover whole diseases when all stages and genetic subtypes are considered.
Gene therapy	A therapy that uses a gene or genes to treat, prevent or cure a disease or medical disorder. Often, gene therapy works by adding new copies of a gene that is defective, or by replacing a defective or missing gene in a patient's cells with a healthy version of that gene.
Health technology	Health technology refers to health products and services, such as pharmaceuticals (including vaccines), highly specialised therapies, diagnostic tests, medical devices, surgically implanted prostheses, medical procedures, digital health technologies and public health interventions.
Health technology assessment (HTA)	Health technology assessments involve a range of processes and mechanisms that use scientific evidence to assess the quality, safety, efficacy, clinical effectiveness and cost-effectiveness of health technologies and services. The purpose of an HTA is to provide policymakers, funders, health professionals and health consumers with the necessary information to understand the benefits and comparative value of health technologies and procedures. This information is then used to inform policy, funding and clinical decisions, and assist with consumer decision-making.
Highly specialised therapies (HSTs)	A category of therapies created under the 2020–25 Addendum to the National Health Reform Agreement regarding HTAs and funding. Medicines and biologicals approved by the Therapeutic Goods Administration and delivered in public hospitals where the therapy and its conditions of use are recommended by the Medical Services Advisory Committee (MSAC) or the Pharmaceutical Benefits Advisory Committee (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 the 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. For the purpose of the Review, HST is intended to refer to cell and gene therapies irrespective of the arrangement where those therapies are ultimately funded.
Incremental cost-effectiveness ratios (ICER)	A comparison of two alternative health technologies calculated by dividing the incremental costs from substituting the proposed

	health technology with its main comparator by the incremental health outcomes from the substitution.
Life Saving Drugs Program (LSDP)	A Commonwealth program that provides fully subsidised access to expensive essential medicines for eligible patients with ultra-rare and life-threatening diseases.
Life Saving Drugs Program Expert Panel (LSDP EP)	The expert panel that considers applications to list new medicines on the LSDP.
Lifecycle of a medicine	Refers to the time period from the development of a medicine, through to being made available to consumers, to termination of market supply.
Medical Services Advisory Committee (MSAC)	The Medical Services Advisory Committee is an independent, expert non-statutory committee that appraises medical services and technologies proposed for public funding and provides advice to the Government on whether a medical service or technology should be publicly funded. It also amends and reviews existing services funded on the Medicare Benefits Schedule or through other programs.
Medicare Benefits Schedule (MBS)	The Medicare Benefits Schedule is a list of health professional services the Australian Government subsidises. MBS items provide patient benefits for a wide range of health services including consultations, diagnostic tests and operations.
National Immunisation Program (NIP)	A Commonwealth funding program that provides free vaccines to eligible people to help reduce diseases that can be prevented by vaccination. The immunisations range from birth through to adulthood. The program provides free essential vaccines to protect eligible people against a range of diseases.
Patient	An individual awaiting or under medical care and treatment.
Patient-relevant outcomes	An umbrella term covering any health outcome that is perceptible to the patient (the more meaningful to the patient, the greater the patient relevance); any resource provided as part of ongoing clinical management of the patient's medical condition, disease or disorder; any working time changes; or any intangible outcome. Common examples of patient-relevant outcomes include primary outcomes, quality-of-life or utility measures, and economic outcomes.
PBAC submission	The submission that a sponsor/pharmaceutical company must make for the PBAC to consider listing a medicine on the PBS.
Person-centred approach	Refers to an approach that treats each person respectfully as an individual human being, and not just as a condition to be treated. It involves seeking out and understanding what is important to the patient, their families, carers, and support people, fostering trust and establishing mutual respect. It also means working together to share decisions and plan care.

Pharmaceutical Benefits Advisory Committee (PBAC)	The independent, expert advisory committee provided for under the National Health Act 1953. Its primary function is to recommend new medicines for listing on the Pharmaceutical Benefits Scheme and vaccines for listing on the National Immunisation Program (NIP). The Pharmaceutical Benefits Advisory Committee is appointed by the Australian Government.
Pharmaceutical Benefits Scheme (PBS)	An Australian Government program that provides Australians subsidised access to a wide range of medicines for most medical conditions.
Post-market review (PMR)	A review that is undertaken at a certain period after a health technology is subsidised. Post-market reviews may be initiated at any time, but the main drivers for a review are recommendations by the Pharmaceutical Benefits Advisory Committee or issues identified through the routine monitoring by the Drug Utilisation Sub-Committee. Routine monitoring occurs at 24 months post-listing for new major listings, and changes to existing listings of medicines on the Pharmaceutical Benefits Scheme (PBS). It is important for the Government to continue to monitor clinical effectiveness and cost-effectiveness of medicines after they have been listed on the PBS. Reviews of cost-effectiveness ensure that the cost of medicines to the PBS appropriately reflects the health outcomes expected and subsequently produced. These reviews are to ensure the quality use of PBS-listed medicines and the ongoing sustainability of the PBS.
Quality use of medicines	A policy objective that seeks to ensure that medicines are used only when needed, choosing suitable medicines and using medicines safely.
Randomised controlled trial (RCT)	An experiment in which investigators randomly allocate eligible people into intervention groups to receive or not to receive one or more interventions that are being compared. The results are assessed by comparing outcomes in the treatment and control groups.
Rare disease	A disease that affects fewer than 5 in 10,000 people.
Reference pricing	Reference pricing is an Australian Government pricing policy where drugs that are considered to be of similar safety and efficacy for pricing purposes are linked and recommended by the PBAC as 'cost-minimised'. The lowest priced drug (or brand) sets a benchmark price for either the other brands of that drug, or the other drugs within the same sub-group of therapeutically related drugs.
Safety	The inverse of toxicity (harm to health caused by a health technology considering the entire profile of adverse reactions and adverse outcomes). Comparative safety is the safety of one health technology compared to an alternative health technology.

	Incremental safety is the absolute difference between the safety profiles of alternate health technologies for the same medical condition, disease or disorder.
Standing Committee on Health, Aged Care and Sport (Standing Committee)	A parliamentary committee that investigates specific matters of policy or government administration or performance in the areas of health, aged care and sport.
Strategic Agreement	An agreement between the Commonwealth and Medicines Australia. The current Strategic Agreement runs from 2022 to 2027. It contains a comprehensive package of reforms to ensure that Australians continue to gain access to breakthrough new medicines as early as possible. The Review is one of the commitments under the Strategic Agreement.
The New Frontier inquiry	The House of Representatives Standing Committee on Health, Aged Care and Sport inquiry into the approval processes for new drugs and novel medical technologies in Australia. This inquiry commenced following a referral on 14 August 2020 from the then Minister for Health. This inquiry has been occasionally referred to colloquially in some external publications as the 'Zimmerman Inquiry'.
Therapeutic Goods Administration (TGA)	The part of the Australian Government Department of Health and Aged Care that regulates the quality, safety and efficacy of therapeutic goods available within Australia.
Ultra-rare disease	A disease that affects no more than 1 person in 50,000.
Uncertainty	Any reduction of confidence in a conclusion. Statistical uncertainty arises from chance (or random variation), when a variable includes a range of estimates within which the true value of the variable is likely to be found. Clinical uncertainty arises when the proposed health technology has both clinical advantages and disadvantages compared with its main comparator(s). Uncertainty also arises when assumptions need to be made in the absence of relevant data.
Unmet clinical need	A condition with no satisfactory method of diagnosis, prevention or treatment.

List of acronyms and abbreviations

Acronym	Full
ABS	Australian Bureau of Statistics
ACCHSs	Aboriginal Community Controlled Health Services
AHTA	Adelaide Health Technology Assessment
AMR	Antimicrobial resistance
ARTG	Australian Register of Therapeutic Goods
ATs	Advanced therapies (see also ATMPs and HSTs)
ATAGI	Australian Technical Advisory Group on Immunisation (Australia)
ATMP	Advanced therapy medicinal products also sometimes known as 'advanced therapies', 'highly specialised technologies', or 'cell and gene therapies'.
CADTH	Canadian Agency for Drugs & Technologies in Health
CAR-T	Chimeric antigen receptor T cell therapy (a type of gene modified cell therapy)
CDC	Australian Centre for Disease Control
CHERE	Centre for Health Economics Research and Evaluation
CO2	Carbon dioxide (Chemical compound)
Consultation Hub	Office of Health Technology Assessment Consultation Hub
DUSC	Drug Utilisation Sub-Committee
ESC (PBAC ESC)	Pharmaceutical Benefits Advisory Committee (PBAC) Economic Sub-Committee. Note: The Medical Services Advisory Committee (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'.
FINOSE	Nordic HTA collaboration arrangement – originally between Finland, Norway and Sweden; now includes Denmark and Iceland.
G7	Group of Seven
HATV	High added therapeutic value
HST	High-cost, Highly Specialised Therapy delivered to public hospital inpatients as defined under the 2020–25 Addendum to the National Health Reform Agreement.
HTA	Health technology assessment
HTAR	Health Technology Assessment Regulation (EU)
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).

KPI	Key performance indicator
LSDP	Life Saving Drugs Program
MAP	Managed Access Program also sometimes known as 'Managed Entry Programs' Note: not to be confused with 'Medicines Access Programs'.
MBS	Medicare Benefits Schedule (Australia)
MCDA	Multiple-criteria decision analysis
MEAs	Managed entry agreements (includes Managed Access Programs (MAPs) and risk-share arrangements (RSAs)).
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
NHRA	National Health Reform Agreement
NICE	National Institute for Health and Care Excellence (UK)
NIHRIO	National Institute for Health and Care Research Innovation Observatory (UK)
NIP	National Immunisation Program (Australia)
NMP	National Medicines Policy
OECD	Organisation for Economic Co-operation and Development
PBAC	Pharmaceutical Benefits Advisory Committee (Australia)
PBS	Pharmaceutical Benefits Scheme (Australia)
PhRMA	Pharmaceutical Research and Manufacturers of America
PICO	Population, Intervention, Comparator, and Outcome
PO	pricing offer
PREM	Patient-reported experience measure
PROM	Patient-reported outcome measure
PSDs	Public summary documents
QALY	Quality-adjusted life year
RCTs	Randomised controlled trials
RPBS	Repatriation Pharmaceutical Benefits Scheme
RWD	Real-world data
RWE	Real-world evidence
SOC	standard of care
STEDI	Spectrum, Transmission, Enablement, Diversity and Insurance Value
TGA	Therapeutic Goods Administration (Australia)
TIS	tisagenlecleucel (a type of CAR-T therapy)
UK	United Kingdom
US	United States of America
WHO	World Health Organization

Reference documents

The following lists some of the documents referred to and used during the Review.

Health Technology Assessment Policy and Methods Review – Terms of Reference

Strategic Agreement 2022-2027

National Medicines Policy

National Agreement on Closing the Gap

Inquiry into approval processes for new drugs and novel medical technologies in Australia

Review of the Discount Rate in the PBAC Guidelines

PBAC Public Summary Document: Review of the base discount rate in the PBAC Guidelines

Health Technology Assessment Policy and Methods Review - Consultation 1 Report

Health Technology Assessment Policy and Methods Review – Consultation 2 Report

Health Technology Assessment Policy and Methods Review – Research and analysis papers:

Adelaide Health Technology Assessment (AHTA)

- Paper 1. International health technology market approval, funding and assessment pathways
- Paper 2. Horizon Scanning and Early Assessment
- Paper 3. HTA Methods: Determination of Population Intervention Comparator Outcome (PICO)
- Paper 4. HTA Methods: Clinical Evaluation

Final versions of Papers 1-4 prepared by AHTA have been consolidated into a single paper: *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*.

Centre for Health Economics Research and Evaluation (CHERE)

- Paper 5. HTA Methods: Economic Evaluation
- Paper 6. Funding and purchasing decisions and managing uncertainty

Centre of Research Excellence in Medicines Intelligence (MI-CRE)

- Paper 7. Optimising the availability and use of real-world data and real-world evidence to support health technology assessment in Australia.

The Department of Health and Aged Care

- Paper 8. Australian market authorisation, funding and assessment pathways and timelines
- Paper 9. Emerging Health Technologies

Terms of Reference

1. Health Technology Assessment (HTA) Policy and Methods Review

1.1. A commitment under the Strategic Agreement

The HTA Review is a commitment in the 2022-2027 Strategic Agreement between the Commonwealth and Medicines Australia (Strategic Agreement) (**Attachment A**). Under clause 5.3 of the Strategic Agreement the Commonwealth agreed to support and resource a HTA Policy and Methods Review (the Review). This commitment is in recognition of the shared goals set out at clause 5.1 of the Strategic Agreement of:

- reducing time to access for Australians so that they can access new health technologies as early as possible
- maintaining the attractiveness of Australia as a first-launch country to build on Australia's status as a world leader in providing access to affordable health care,

by ensuring that our assessment processes keep pace with rapid advances in health technology and barriers to access are minimised.

1.2. HTA Review process

Under Clause 5.3.1 of the Strategic Agreement, it was agreed that a Reference Committee would be established and would include an Independent Chair, the Chair of the Pharmaceutical Benefits Advisory Committee (PBAC), a Government nominee, a member nominated by Medicines Australia and a patient representative. It was subsequently agreed that the Reference Committee would be expanded to include two patient representatives and a clinical / scientific representative. The Minister for Health and Aged Care also agreed to extend the deadline for the HTA Review to 31 December 2023.

Under clause 5.3 of the Strategic Agreement, it was agreed that the Reference Committee would:

1. develop the Terms of Reference for the HTA Review, in consultation with the PBAC and other stakeholders including Medicines Australia
2. 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 jurisdictions guided by the Terms of Reference
3. oversee public consultations and consider submissions to the HTA Review
4. oversee the analysis undertaken by the expert in HTA and

5. prepare and agree the final report and recommendations to the PBAC and the Commonwealth.

Under clause 5.4 of the Strategic Agreement, it was agreed that the final report of the Reference Committee, including recommendations, will be provided to the PBAC (and its technical subcommittees) and the Commonwealth for consideration by the Australian Government.

2. Context

2.1. Australia's National Medicines Policy

The vision of the National Medicines Policy (NMP) is to achieve the world's best health, social and economic outcomes for all Australians through a highly supportive medicines policy environment.

The aim of the NMP is to ensure:

- Equitable, timely, safe and affordable access to a high-quality and reliable supply of medicines and medicines-related services for all Australians.
- Medicines are used safely, optimally and judiciously, with a focus on informed choice and well-coordinated person-centred care.
- Support for a positive and sustainable policy environment to drive world-class innovation and research, including translational research, and the successful development of medicines and medicines-related services in Australia.

2.2. HTA in Australia

The NMP vision and aims are supported by subsidy schemes and funding programs like the Pharmaceutical Benefits Scheme (PBS), the Medicare Benefits Schedule, the National Immunisation Program and the Life Saving Drugs Program (LSDP) and through the National Health Reform Agreement between the Australian Government and all state and territory governments. These programs have, over many years, enabled Australians to gain subsidised access to the most effective health technologies for the prevention, management, and treatment of medical conditions. The purpose of these programs is ensuring Australians have access to the treatments that they need. The processes of acquiring these medicines by necessity involves commercial negotiations and arrangements between the suppliers and the Australian Government.

To ensure value for the expenditure of public funds, an essential step in Government decisions to subsidise health technologies involves advice from independent expert committees comprising doctors, health professionals, health economists and consumer representatives. These members are appointed to be the pre-eminent source of advice to Government on decisions to subsidise health technologies (including for whom and at what cost). When deciding their advice, the expert advisory committees consider an evaluation which summarises relevant information including clinical safety, effectiveness and cost of health technologies compared to alternatives and a range of

other factors. HTA enables recommendations to Government that 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 sponsor.

Introduction of new health technologies typically requires new government expenditure in order to purchase proprietary products from commercial suppliers (sponsors). Ultimately there will always be tension on cost of a product between a commercial supplier seeking reward for their innovation in bringing the product to market and a sensible buyer, seeking value for their money. In this instance, the buyer is the Government acting on behalf of all Australians.

The PBAC decision-making, for example, is influenced by five quantitative factors:

1. comparative health gain assessed in terms of both the magnitude of effect and clinical importance of effect
2. comparative cost-effectiveness presented as incremental cost-effectiveness ratios (including incremental cost-utility ratios) or a cost-minimisation approach
3. affordability in the absence of PBS subsidy
4. predicted use in practice and financial implications for the PBS
5. predicted use in practice and financial implications for the Government's health budget.

Other less-readily quantifiable factors that also influence PBAC decision-making include:

1. overall confidence in the evidence and assumptions relied on in submissions
2. equity of access issues such as age, or socioeconomic and geographical status
3. presence of effective therapeutic alternatives where it influences the need for the medicine on the PBS
4. severity of the medical condition treated, emphasising the nature and extent of disease as it is currently managed
5. ability to target therapy with the proposed medicine precisely and effectively to patients likely to benefit most
6. public health issues such as the development of antimicrobial resistance
7. any other relevant factors that may affect the suitability of the medicine for listing on the PBS instead of other Government programs that support healthcare access
8. consumer comments, which help the PBAC understand what consumers consider to be the main benefits and harms of the proposed medicine.

In special circumstances of high unmet clinical need, there are also managed access arrangements that enable subsidy of some new health technologies on terms that allow for the resolution of otherwise unacceptable clinical or economic uncertainty.

Formal HTA is an approach to ensure these factors are considered in a consistent way. HTA methods continuously evolve, necessitating periodic review and update of HTA

policy and methods. Since the requirement for the PBAC to consider cost-effectiveness in its decisions in 1987, the PBAC guidelines on submissions have been reviewed at regular intervals – most recently in 2016. The Medical Services Advisory Committee (MSAC) guidelines have also been reviewed periodically since 1998.

2.3. How the HTA Review fits with recent medicine reform processes

Recently, both the Standing Committee on Health, Aged Care and Sport (Standing Committee) inquiry into approval processes for new drugs and novel medical technologies in Australia (the Inquiry) and the NMP Review heard a range of views about the new types of health technologies that are emerging and the changing expectations of Australians including where they are not currently being met by Australia's subsidy schemes and funding programs. Under the direction set by the Strategic Agreement, the Inquiry and the new NMP, the HTA Review is an important opportunity to develop specific reforms to how health technologies are assessed and funded to help ensure that Australia's subsidy schemes and funding programs continue to meet the needs of Australians into the future.

The Standing Committee on Health, Aged Care and Sport Inquiry into approval processes for new drugs and novel medical technologies in Australia

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.

The HTA Review will address the issues identified in the Inquiry, and the recommendations of the Standing Committee, while also recognising that there are several HTA reform processes that are being undertaken in parallel to the HTA Review (section 5).

National Medicines Policy

The central pillars of the new NMP are:

- equitable, timely, safe and reliable access to medicines and medicines-related services, at a cost that individuals and the community can afford
- medicines meet the required standards of quality, safety and efficacy
- quality use of medicines and medicines safety
- collaborative, innovative and sustainable medicines industry and research sectors with the capability, capacity and expertise to respond to current and future health needs.

The new NMP also identifies a set of fundamental principles to guide partners in achieving the NMP's aim. These fundamental principles are: person-centred, equity and access, partnership based and share responsibility, accountability and transparency, innovation and continuous improvement, evidence based, and sustainability.

The HTA Review will seek to further the objectives of the NMP to ensure that Australia's subsidy schemes and funding programs continue to deliver the best possible access for Australians to the treatments they need.

3. HTA Review objectives

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 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 and
7. do not compromise assessment of patient safety, effectiveness and cost, or advice to Government on subsidy of health technologies.

4. HTA Review Terms of Reference

4.1. Health Technologies

HTA policy and methods for the following health technologies will be considered by the HTA Review:

1. all medicines and vaccines
2. highly specialised therapies (such as cell and gene therapies)
3. other health technologies (for example a pathology test or an imaging technology) that improve health outcomes associated with the technologies defined in points 1 and 2
4. foreseeable changes in health care that may influence the need, accessibility, effectiveness or cost-effectiveness of new health technologies.

4.2. Policies and methods

The HTA Review will examine Commonwealth HTA policy and methods (including those set out in the PBAC and MSAC Guidelines where applicable to the technologies outlined in Section 4.1) relating to:

1. identification of place of a technology in care and selection of comparators
2. identification of patient relevant outcomes
3. augmentation of primary clinical evidence with data designed to capture the value of health technologies from the perspective of patients and their communities (such as qualitative research, patient preference studies, patient reported outcome measures and patient reported experience measures)
4. evaluations (including how the value of medicines is captured)
5. incorporation and use of direct input from patients, clinicians and other stakeholders with professional or lived expertise, into HTA evaluations and deliberations
6. approaches to increasing transparency in HTA decision-making and communicating this
7. new technologies, or expanded indications, that provide a substantial improvement in health outcomes compared to relevant alternative therapies
8. new technologies, or expanded indications, that do not provide a substantial improvement in health outcomes compared to relevant alternative therapies
9. managing clinical, economic, financial, and other uncertainty throughout the lifecycle of a technology including better capture of necessary data on duration of effectiveness and safety events and
10. assessment of technologies (such as those for rare and ultra-rare diseases) that would be used for conditions where there is high unmet clinical need that have clinical and economic uncertainty including:

a. use of evidence from relevant sources other than randomised controlled trials where such trials are not feasible and
b. arrangements for post market assessment and decision-making.

4.3. Funding and approval pathways

The HTA Review will consider efficient and equitable assessment and funding approaches and pathways in relation to the technologies at 4.1. This discussion will include:

1. approaches that incentivise launch of first in class technologies or first major extension of indication that deliver a substantial improvement in health outcomes compared to relevant alternative therapies
2. equitable distribution and efficient use of limited HTA resources to meet the health and wellbeing needs of the Australian population
3. implications of any recommendations for assessment of other health technologies and hospital funding
4. management of future advances in health care including:
   a. adaptability of HTA approaches
   b. flexibility of advisory committee decision-making
   c. avoiding unnecessary complexity or duplication in HTA.
5. consideration of equity of access in HTA decision-making including for the following groups:
   a. First Nations people
   b. people from culturally and linguistically diverse backgrounds
   c. children and older people
   d. people with disability
   e. people living in rural and remote areas
   f. people of low socioeconomic status
   g. people living with rare and under-recognised diseases
   h. people with mental illness
   i. lesbian, gay, bisexual, transgender, queer or questioning, intersex and/or other sexuality and gender diverse people (LGBTQI+)
6. other populations in circumstances and at life stages that give rise to vulnerability.
   a. the feasibility of international work sharing for evaluation of technologies in scope for the HTA Review
   b. purchasing practices used by comparable international jurisdictions.

5. Concurrent HTA reform processes

There are several reform processes to HTA that are being undertaken in parallel to the HTA Review. The Reference Committee will work closely with areas undertaking these processes to ensure it is informed by what is learnt through, and the HTA Review recommendations are aligned to the outcomes of, those processes.

5.1. Processes for patient and consumer engagement

The Government is undertaking several reform activities that seek to improve the way patients, consumers and carers are engaged and included in HTA. This includes Conversations for Change community consultations which aim to explore different options and approaches to improve communication and engagement and to better support consumers, patients and carers during the HTA process. The findings of these consultations will be collated and analysed so the key priorities of everyone involved in the consultations will be understood, and proposals can be developed. These key priorities will be used to inform other reforms including the commitment under clause 6.3 of the Strategic Agreement to co-design an Enhanced Consumer Engagement Process to capture consumer voices for applications to list new medicines on the PBS. The Enhanced Consumer Engagement Process is intended to facilitate the capture of informed consumer and patient perspectives earlier, to effectively inform the assessment of submissions for reimbursement of innovative medicines and subsequent consideration by the PBAC.

5.2. Expertise, role, and remit of advisory committees

The expertise of advisory committees was examined in, and the subject of a recommendation from the Inquiry. The Standing Committee recommended that:

> *the Australian Government ensure the membership of the Pharmaceutical Benefits Advisory Committee and Medical Services Advisory Committee provides the appropriate expertise for all applications. This should include the possibilities of enhanced cross-membership between the two committees and the appointment of temporary members to consider individual applications. Recognising the nature of health challenges in Indigenous communities, membership should include representation from Aboriginal and Torres Strait Islander Peoples.*

This matter will be considered as part of the Government's response to the Standing Committee recommendations. The HTA Review will consider matters of committee organisation and processes that relate to the efficiency and timeliness of HTA considerations and subsequent decision-making.

5.3. International Collaboration Arrangement between the Department of Health and Aged Care and other health technology assessment bodies

The Department of Health and Aged Care has signed an international collaboration arrangement with health technology assessment bodies internationally. The signatories to the arrangement, who will continue to remain independent of one another, are:

- Australian Government Department of Health and Aged Care
- National Institute for Health and Care Excellence (NICE)
- Canadian Agency for Drugs and Technologies in Health (CADTH)
- Healthcare Improvement Scotland
- Health Technology Wales
- All Wales Therapeutics & Toxicology Centre
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5.4. Other HTA reform commitments under the Strategic Agreement

The Strategic Agreement contains several additional commitments to reform of HTA processes. This includes:

a. continuous process improvement to HTA processes to facilitate earlier access to medicines
b. consideration of options for conditional funding arrangements that complement the priority and provisional medicine pathways used by the TGA
c. co-design of a trial to facilitate exchange of information between sponsors and evaluators during the process of a particular PBAC submission
d. the development of a policy for Risk Sharing Arrangements and
e. rapid post-market reviews.

6. Areas that are out of scope for the HTA Review

6.1 Government health and economic decision-making

The Government has agreed to funding parameters that allow the Minister for Health and Aged Care to approve the PBS listing of a new medicine up to $20M in any year. Beyond this cost, the PBS listing would require Cabinet approval. The Government has given a high priority to funding new medicines recommended by the PBAC. It can do this because the processes of the PBAC ensure value for spending on medicines. However, this decision-making occurs in the broader context of the Government and Cabinet health and economic decision-making. This broader policy setting and decision-making is outside the terms of reference for this review.

Consultation scope

Terms of reference

The terms of reference for the HTA Review 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 Review was seeking to identify, as well as objectives for the final recommendations.

Public Consultation 1

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

Consultation 1 received 114 submissions, which included responses to the online survey through the Office of Health Technology Assessment Consultation Hub (Consultation Hub), emailed submissions, and online video forums with the Reference Committee.

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

- Paper 2. Horizon Scanning and Early Assessment
- Paper 3. HTA Methods: Determination of Population Intervention Comparator Outcome (PICO)
- Paper 4. HTA Methods: Clinical Evaluation

Final versions of Papers 1-4 prepared by AHTA have been consolidated into a single paper: *HTA Pathways and Processes, Clinical Evaluation Methods and Horizon Scanning*.

Centre for Health Economics Research and Evaluation (CHERE) papers include:

- Paper 5. HTA Methods: Economic Evaluation
- Paper 6. Funding and purchasing decisions and managing uncertainty

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.

Options paper

The Reference Committee considered stakeholder feedback alongside expert input and extensive research to develop an Options Paper, which was published on 25 January 2024. The Options Paper presented an overview of the current state, what the Reference Committee heard from stakeholders, identification of the issues, and the options for reform being considered by the Reference Committee to improve Australia's HTA policies and methods and the funding and approval pathways.

The Options Paper was workshopped with stakeholders through the second round of public consultation (Consultation 2).

Public Consultation 2

Consultation 2 was open from 25 January 2024 to 23 February 2024, and consisted of:

a. An online consultation survey made available to stakeholders on 6 February 2024.
b. Online workshops held on 13, 15 and 16 February 2024.
c. An in-person workshop held in Sydney on 19 February 2024.

Consultation 2 received 139 written submissions and additional feedback.

List of tables and figures

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