

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

Recommendations summary



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](#)



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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

** 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.*

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.

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.

Context

The Health Technology Assessment Policy and Methods Review (Review) was established to examine Australia's approach to assessing health technologies for government funding, and to deliver a report and recommendations to the Australian Government.

The Review has recommended comprehensive reforms that take a multifaceted approach to improving Australia's health technology assessment (HTA) arrangements. This includes reforming HTA policy and methods to provide stakeholders and decision-makers with tools and processes to:

- address inequities in access to affordable medicines
- reduce wait times for access to affordable medications
- improve transparency and engagement
- invest in HTA capability to make it adaptable and futureproof.

The reforms are comprehensive. They range from horizon scanning, planning and system readiness to proactively bringing important medicines and vaccines to Australia. The central tenet of the Review, reflected through extensive engagement with the Australian community, is that it is vital to improve access to affordable medicines and vaccines to meet the needs of all Australians, particularly First Nations people.

The full report of the Review: [*Accelerating Access to the Best Medicines for Australians Now and into the Future*](#) is available.

Included in this summary paper is a brief overview of the full report including a summary of the key recommendations and their objectives.

What HTA is and why it is important

Australia's health outcomes are among the best in the world and continue to improve.¹ Our universal health system contributes to these health outcomes through providing access to high-quality, safe, effective and appropriate health care at low or no cost to citizens. This includes subsidised access to health technologies such as medicines, vaccines, medical devices and health services.

To achieve these 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.

¹ OECD (2023) [*Health at a Glance*](#).

HTA processes are vital for deciding which health technologies should be made available under our healthcare schemes. They involve evaluating scientific evidence to determine the quality, safety, efficacy, cost-effectiveness and total costs of health technologies compared to the existing standard of care for patients.

For Australian citizens to benefit from the latest health technologies, decision-makers need to be able to conclude, in a timely way, that each technology will work as well as, or better than, what is already available, and that we will be better off if they are funded.

HTA policies and processes are fundamental to the sustainability of Australia's universal healthcare system and ensuring that citizens have access to the most effective health technologies to prevent, manage and treat medical conditions.

Why Australia's approach to HTAs is being reviewed

Health technologies have been a major contributor to improving healthy life expectancy over the past century. Some of the latest technologies could greatly improve and extend the lives of people living with debilitating and/or life-shortening conditions, or treat lethal or severely disabling conditions that couldn't be effectively treated in the past.

Making decisions about funding and providing access to new and emerging health technologies is increasingly challenging. Health technologies are becoming more complex and diverse. And many do not neatly fit the treatment categories under Australia's funding and assessment processes.² Additionally, the evidence needed to conclude how well emerging health technologies, such as cell and gene therapies, are likely to perform means decision-makers aren't as confident as they were about medicines that emerged in the 1990s and 2000s. Health technologies are also becoming increasingly expensive. The most expensive medicine on the Pharmaceutical Benefits Scheme (PBS) costs a little over \$2.5 million, 100 times the price of the most expensive treatment listed in 2010.

The system that advises the Australian Government about which health technologies should be funded, for whom, and under what circumstances, needs to be able to respond to rapid advances in medical technologies. Patients, clinicians, industry and the Government have expressed concern that funding new health technologies takes too long and that HTA processes need to be futureproofed for emerging technologies.

² See p11–12 in Department of Health and Aged Care (DHAC) (2023) [Emerging health technologies](#), Health Technology Assessment Policy and Methods Review.

These issues have been raised in other inquiries and reform processes, including the *2022–2027 Strategic Agreement between the Commonwealth and Medicines Australia*,³ the NMP Review⁴ and 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).⁵

The Review presented a significant opportunity to examine Australia’s approach to HTAs and recommend implementable reforms to the Australian Government.

How the Review was conducted

The Minister for Health and Aged Care appointed the Reference Committee to conduct the HTA Review to ‘keep HTA in Australia at the forefront of public health and ensure Australians continue to enjoy the best possible access to health technologies, at a cost consumers and the public can afford’.⁶

The Reference Committee developed the Review’s Terms of Reference, met regularly and were open and inclusive with stakeholders throughout the process.

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:

- are working effectively
- may act as current or future barriers to earliest possible access
- may act as current or future barriers to equitable access
- detract from person-centredness
- may be creating perverse incentives..

The Review aimed to create an implementable, sustainable set of recommendations to address identified challenges and provide Australians with equitable, timely and safe access to affordable medicines. It also aimed to ensure that HTA policy and methods were equipped to evaluate emerging technologies in the future.

The Reference Committee delivered its findings and recommendations after considering extensive evidence and inputs. This included conducting comprehensive consultations and commissioning HTA experts to develop papers on contemporary

³ DHAC (2022) [Strategic Agreement between the Commonwealth of Australia and Medicines Australia 2022-2027](#).

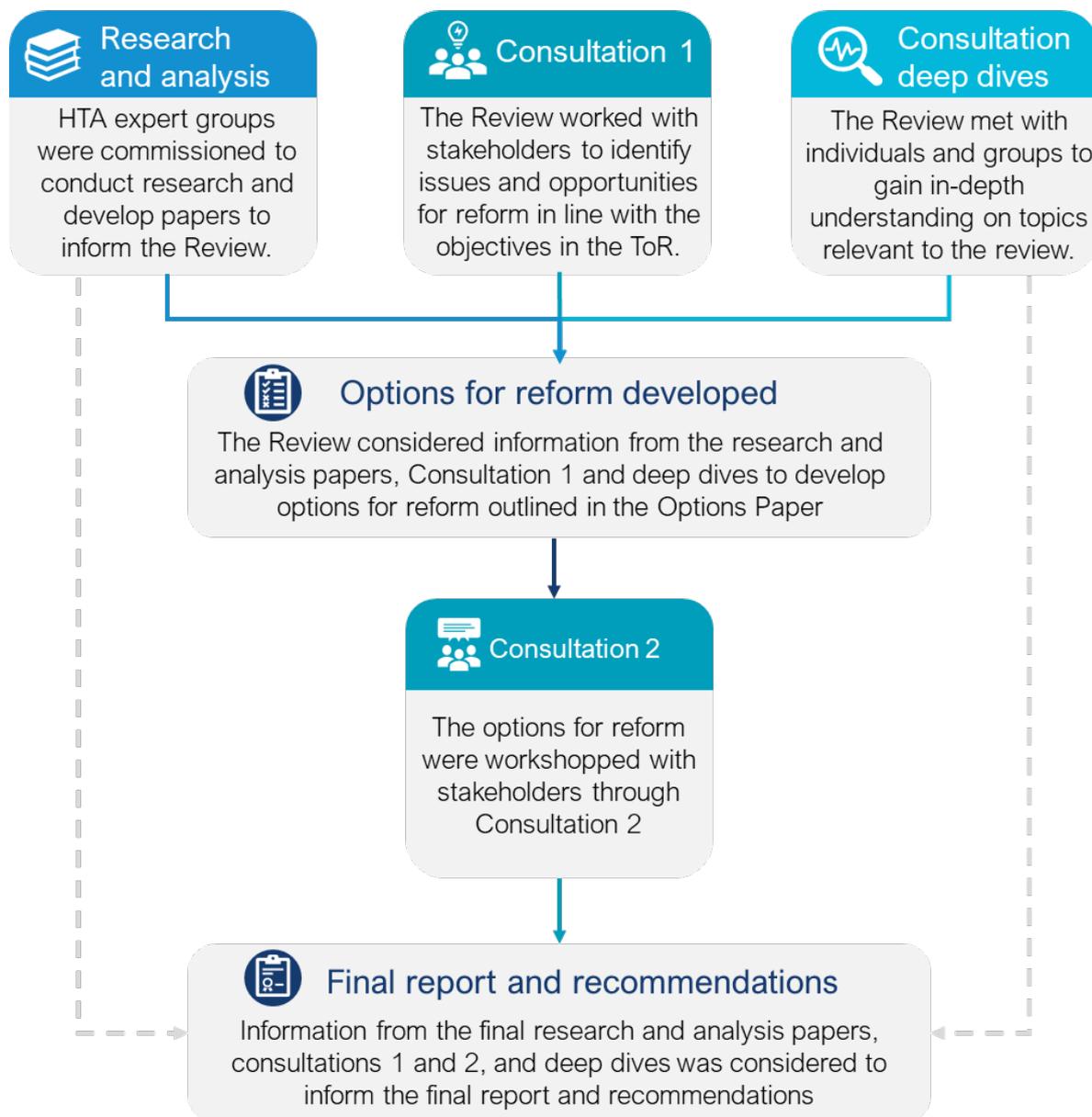
⁴ DHAC (2022) [National Medicines Policy - consultation on the revised NMP](#), DHAC Consultation Hub DHAC website.

⁵ Australian Parliament House of Representatives Standing Committee on Health, Aged Care and Sport (2021) [The New Frontier inquiry](#).

⁶ DHAC (2022) [HTA review and chair committee](#) [media release].

research, and relevant methodologies and purchasing practices in comparable international jurisdictions.

The Review process



Overview

This report provides a brief overview of the full report: *Accelerating Access to the Best Medicines for Australians Now and Into the Future*, including a summary of the key recommendations and objectives. To read all the recommendations in full detail, and the findings and analysis they are based on, please see the full report. Chapters 1 and

2 of that report provides an introduction and overview of Australia's health system, HTA processes and the Review. The other chapters look at:

- improving equitable access to health technologies for under-represented patient groups
- streamlining HTA pathways and processes to improve the time for patients to assess health technologies
- improving policies, methods and processes for translating HTA recommendations into patient access to health technologies
- improving transparency and stakeholder involvement
- enhancing real-world data and real-world evidence for HTAs
- enhancing HTA methods so that decision-makers can be confident health technologies will perform as claimed.
- improving the processes and structures that support the HTA system.

Each chapter in the full report introduces the topic; considers the issues and opportunities for reform, including what we heard from stakeholders through consultations; and presents the Review's findings and recommendations. The recommendations are accompanied by an objective, explaining what the recommendations are intended to achieve.

This document summarises the full report's key recommendations and related objectives.

Summary recommendations and objectives

The following provides a summary of the Review's key recommendations for the Australian Government to action in collaboration with relevant stakeholders. The full, detailed recommendations as well as the Review's consideration of issues, analysis and findings are contained in the relevant chapters of the full report.

A [list of the full recommendations](#) is available.

A Glossary of commonly used terms and phrases noted in this report is available at the Glossary section of [the full report](#).

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

Chapter 3.1: Creating an equitable system for First Nations people

Objectives

The recommendations in this chapter are intended 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.

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

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.

Chapter 3.2: Improving access to medicines for paediatric patients

Objectives

The recommendations in this chapter are intended to improve access to PBS-listed medicines for paediatric patients and facilitate regulatory approval and reimbursement of medicines for paediatric indications.

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

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

Objectives

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.

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. Triage 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.

Chapter 4.2: Improved pathways for listing medicines on the PBS

Objectives

The recommendations in this chapter are intended to:

- reduce the time and effort that sponsors, the Department, evaluators and the (Pharmaceutical Benefits Advisory Committee) 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 high added therapeutic value.

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.

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

Objectives

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.

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.

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

Objectives

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

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 HTAs as outlined in Schedule C of the 2020–25 Addendum to the National Health Reform Agreement.⁷

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.

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

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

Objectives

The recommendations in this chapter 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 Life Saving Drugs Program, and subsequent ongoing management arrangements, are clearly stated in relevant guidance and policy

⁷ DHAC (2020) [2020–25 Addendum to the National Health Reform Agreement](#).

- 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.

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.

Chapter 4.6: Measuring the impact of reforms on timeliness

Objectives

The recommendations in this chapter 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 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 Australian Register of Therapeutic Goods (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

Chapter 5.1: Alternative ways to pay for health technologies

Objectives

The recommendations in this chapter are intended to 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.

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.

Chapter 5.2: Improving the post-HTA negotiation process

Objectives

The recommendations in this chapter 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.

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.

Chapter 5.3: The need for regular reassessment of health technologies

Objectives

The recommendations in this chapter 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.

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.

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

Objectives

The recommendations in this chapter 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.

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.

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

Objectives

The recommendations in this chapter seek implementation of 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.

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

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

Objectives

The recommendations in this chapter are intended to 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.

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.

Chapter 6.2: Consumer, clinical and other stakeholder involvement and consideration in HTAs

Objectives

The recommendations in this chapter are intended to improve communication, ensure transparency and stakeholder engagement are multifaceted and person-centred, and include short-, 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.

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.

Chapter 6.3: Development of an explicit qualitative values framework

Objectives

The recommendations in this chapter are intended 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.

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 HTAs

Chapter 7.1: Optimising real-world data and real-world evidence for HTAs

Objectives

The recommendations in this chapter are intended to enhance timely access to relevant, quality real-world data and real-world evidence 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 randomised controlled trial 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.

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

Chapter 8.1: Population, Intervention, Comparator, and Outcome

Objectives

The recommendations in this chapter are intended to put patients at the centre of the development of the Population, Intervention, Comparator, and Outcome (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.

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.

Chapter 8.2: Use of consumer evidence and input

Objectives

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 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: Transparency and stakeholder involvement) into HTA processes.

Chapter 8.3: Clinical evaluation

Objectives

The recommendations in this chapter are intended 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.

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.

Chapter 8.4: Economic evaluation

Objectives

The recommendations in this chapter seek 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 Pharmaceutical Benefits Advisory Committee 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 Pharmaceutical Benefits Advisory Committee and Medical Services Advisory Committee 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.

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.

Chapter 8.5: Environmental considerations

Objectives

The recommendations in this chapter aim to set out new arrangements that recognise the substantial research and consultation conducted during the development of the National Health and Climate Strategy⁸ 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.

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

Chapter 9.1: Proactively addressing areas of unmet clinical need

Objectives

The recommendations in this chapter aim to set out new arrangements that focus on identifying, screening and inviting the submission of health technologies for public subsidy that address high unmet clinical need and health inequities.

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.

⁸ DHAC (2023) [National Health and Climate Strategy](#).

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.

Chapter 9.2: Horizon scanning

Objectives

The recommendations in this chapter aim to 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.

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.

Chapter 9.3: Mechanisms for continuous review and improvement

Objectives

The recommendations in this chapter seek to 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.

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.

Chapter 9.4: Capacity and capability of the HTA system and supporting architecture

Objectives

The recommendations in this chapter seek to emphasise the importance of having a 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 National Medicines Policy pillars and principles.⁹

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.

⁹ DHAC (2022) [National Medicines Policy](#).