Health Technology Assessment Policy and Methods Review

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 healthcare,

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 preeminent 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 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 health care 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.

2.3.1 *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).

2.3.2 *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+)
    j. other populations in circumstances and at life stages that give rise to vulnerability.
6. the feasibility of international work sharing for evaluation of technologies in scope for the HTA Review
7. 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 of an Enhanced Consumer Engagement Process to capture consumer voices in respect of 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

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. 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.