February 2024

Medicines Australia's Submission to the Health Technology Assessment Policy and Methods Review – Consultation 2

An Opportunity for Transformative Reform

Medicines Australia

Executive Summary

The Health Technology Assessment (HTA) Policy and Methods Review is in the national interest and is crucial for fulfilling the vision in the *National Medicines Policy* (NMP) to achieve the world's best health, social and economic outcomes for all Australians. It is a key priority of Medicines Australia's *Strategic Agreement* with the Commonwealth and also supports three of the five themes from *Measuring What Matters: Australia's First Wellbeing Framework*: 'healthy', 'sustainable' and 'prosperous'. Medicines Australia welcomes the development of the *Options Paper* and acknowledges the work of the Reference Committee throughout the Review. The paper captures most of the issues and includes many of the recommendations that have been put forward by stakeholders, including those in Medicines Australia's *HTA Roadmap of Reforms*; however, some proposals will not be appropriate for the final report. This response will provide feedback on these. Many of the options represent an opportunity for transformative reform of our HTA system, something that is crucial if Australian patients are to benefit from future innovative medicines.

There is widespread recognition, seen through hundreds of submissions to the current Review and previous inquiries, that appropriately recognising the value of medicines and vaccines (including the broader social and economic benefits) is key to making them available to Australian patients as soon as possible. This requires renewed investment. As such, the options that ultimately flow through to the final recommendations and subsequently to implementation (once agreed by Government) must be those that will deliver on the goals of the Review: to reduce time to access for Australian patients, and maintain the attractiveness of Australia as a first-launch country. Options that focus on value recognition and creating process efficiencies will support these goals (in fact, process efficiencies must be supported by value recognition to be effective), whereas options that focus on cost-cutting measures will undermine them.

No single option will achieve the necessary reforms on its own. The options that Medicines Australia support work together synergistically and they all need to be implemented appropriately to achieve the desired outcomes.

When it comes to implementation, a phased approach will be required, and it will be essential for the priorities and details to be discussed and negotiated with all stakeholders in a collaborative, co-design approach, otherwise the resulting policies, processes and methods will not deliver for Australians. There must be a clear commitment to implementation timeframes and measuring the impact of the options that are implemented, so that reforms aimed at delivering faster access to new medicines do in fact result in a tangible improvement for patients as soon as possible.

A such, the Reference Committee's final report to Government must contain recommendations on how implementation should be taken forward, with timelines for implementation. This will provide some confidence and accountability to all those who have contributed to this and other similar reviews.

The HTA reforms must ensure that there is faster, earlier, better access for patients across the board, whether it is for breakthrough medicines addressing a high unmet clinical need (HUCN), medicines using the cost-effectiveness pathway or medicines using the cost-minimisation pathway. The HTA reforms must also be implemented in such a way that they ensure the future sustainability of the medicines industry whilst ensuring value for money for Australian society.

Medicines Australia has viewed all the options through the lenses of faster access, appropriate value recognition, and patient and clinician choice. Our vision is that Australia is a country where new

medicines are launched early, and that all Australians have access to the latest medical technologies within 60 days of TGA registration. As such, we support, in principle, many of the options presented (recognising that detail is limited and a great deal of work needs to be done to ensure implementation delivers on the intent) and oppose those that will undermine the objectives of the Review.

There are five options that increase barriers or are detrimental to patient access, fail to recognise value and choice, are not viable for the industry, do not address the identified issues, or are outside the terms of reference for the Review. These are summarised in the table below.

There are also a number of options that must be strengthened or amended to better deliver the intended outcomes, and these are outlined in the relevant sections.

We have structured our submission around the three feedback questions, with a chapter each on:

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

We have also completed the online survey.

The innovative medicines industry has partnered with governments over decades to ensure the continued reimbursement of new medicines, so that Australians have access to the very best treatments. The industry has delivered billions of dollars in budget savings through successive Strategic Agreements that are forever embedded in the system. The savings include: 2010 ($1.9 billion), 2015 ($6.5 billion), 2017 ($1.8 billion) and 2022 ($1.9 billion). These savings were intended to deliver agreed policy initiatives including the current HTA Review and resulting reforms, which must now be funded. Furthermore, PBS expenditure has shrunk as a proportion of healthcare expenditure from 20% to 17%[1], despite significant improvements to mortality and morbidity over that period.

Australia's population continues to grow and age, and science continues to deliver breakthrough treatments for patients. This makes it imperative that we deliver significant reform through this review. It is time for fundamental HTA reform to ensure its future sustainability so that it can deliver the world's best health, social and economic outcomes for all Australians.

The biopharmaceutical industry has been identified as a key pillar for Australia's economic recovery. In this regard, Medicines Australia believes the area which has not been specifically addressed in the Options Paper is securing Australia's position in the global context, as a first launch country. The HTA system affects the attractiveness of Australia as a first launch market through how medicines are valued, particularly the recognition of, and investment in the innovation inherent in new medicines. Proper recognition of the broad value of individual innovative medicines needs to be a commitment delivered through the various HTA reforms which are implemented.

Punitive savings measures that increase barriers to entry and undervalue the industry's contribution to Australia's health and economic wellbeing will see Australia further slip down the ranks of investment-worthy countries, or even become a country where innovative medicines are not launched at all, with the consequence that patients, clinicians and overall prosperity will suffer.

[1] https://www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2023/06/Funding-Innovative-Medicines-1.pdf

Investment in medicines delivers better outcomes for patients, productivity gains for the Australian economy and encourages a thriving biopharmaceutical industry. Medicines Australia looks forward to working with the Government, patients, carers and clinicians on transformative reforms in the interests of faster, more equitable access for patients.

Should the Reference Committee have any questions about this submission, please do not hesitate to get in touch. Inquiries can be directed to Anne-Maree Englund (Head of Strategic Policy Implementation) at anne-maree.englund@medicinesaustralia.com.au.

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CEO Medicines Australia

Options which industry opposes or which require amendment

Medicines Australia has serious concerns about the following options as they will ultimately have negative consequences for patients and industry through slowing down access, limiting choice, and disincentivising sponsors from launching medicines in Australia.

Option 4.1 Approaches to funding or purchasing new health technologies: recognising competition between new health technologies that deliver similar outcomes: 'require offers of a lower price' or 'incentivise offers of a lower price'

- This option must not appear in the Reference Committee's final report. Price-reduction measures are outside the terms of reference for the Review, and this option would lead to fewer medicines coming to market and less choice for patients and clinicians. In combination with the lowest cost comparator approach this is unreasonable and unfeasible. It also undermines the shared principles in the *Strategic Agreement* of timely access to innovative F1 medicines (E.4), and acknowledgement of the value of the innovator pharmaceutical industry to ensure a healthy Australia (E.6).

Option 3.3 Economic Evaluation: selection of the comparator

- This option must be significantly strengthened with legislative change, otherwise it will not improve value recognition and will undermine the potentially positive outcomes of other options. Lowest cost comparator including F2 medicines must be reconsidered.

Option 3.3 Economic Evaluation: valuing of long-term benefits

- This option must be significantly strengthened with an immediate reduction in the discount rate in line with comparable jurisdictions, otherwise it will not improve value recognition and will undermine the potentially positive outcomes of other options. This is especially relevant for vaccines, preventions or treatments given early in life.

Option 4.1 Approaches to funding or purchasing new health technologies: post-listing reassessment of health technologies

- This option must not appear in the Reference Committee's final report. There is already a post-market review framework which was recently updated in consultation with industry and other stakeholders. In addition, the series of statutory price reductions, and price disclosure, to which industry agreed to create headroom for new medicines, essentially reduces value throughout the lifecycle of a product. In Medicines Australia's view, this represents an existing disinvestment approach. No new framework is needed. It also undermines the shared principle in the *Strategic Agreement* of stability and certainty for investment in innovative medicines (E.2).

Option 5.6 Strengthen international partnerships and work-sharing: collaboration with international jurisdictions to deliver sustainable access to health technologies

- This option must not appear in the Reference Committee's final report as it could slow access even further and become a barrier to access occurring at all. MA opposes international collaboration in HTA being used to establish multi-country buying blocs and pricing controls. It also undermines the shared principle in the *Strategic Agreement* of transparency, predictability and efficiency of processes for listing medicines on the PBS (E.4).

In addition to the above, there are a number of other options which Medicines Australia supports in principle but which must be strengthened or amended (as in their current form they will not address the issues as intended) or which are not required. These are described in detail in Chapter 1.

Overarching Recommendation

The Reference Committee must make recommendations to Government on how implementation of HTA reform should be taken forward, including:

- an appropriate governance structure, reporting to the Minister for Health
- processes for broad stakeholder engagement and collaborative co-design of reforms
- phased implementation over a three-year timeframe with meaningful milestones
- transparent reporting on progress, including agreed KPIs on the aims of the reform (time to access, first-launch country and value recognition).

Q1 Will the proposed options achieve the intended outcome?

Overall, Medicines Australia believes that many of the proposed options will go some way to achieving the intended outcome, but only if they are approached as a package of integrated reforms. The options presented are high level, and there is insufficient detail to understand how they will work in practice. To ensure there are no unintended consequences, implementation will require all relevant stakeholders to formally collaborate on the options and transparently co-design the necessary policies, methods and processes.

There are five options that are detrimental to patient access, undermine value and choice, are not viable, do not address the issues or are outside the terms of reference for the review.

There are also a number of options that will only partially meet the outcomes and must be amended or strengthened. These are discussed in more detail below.

It is important to emphasise that there are many features of the current system that are working well and should be retained (and in some cases enhanced) including: special pricing arrangements and confidential pricing, indication-based pricing, parallel processing, defined timelines, the PBS process improvements, flexibility in decision making, transparency of process, the TGA reforms, patient and prescriber choice, and deeds of agreement.

Medicines Australia would like to make the general comment that reforms must be appropriately funded by Government. Any consideration of cost recovery for the proposed options must be done in consultation with industry, and that there should be appropriate fee waivers for certain categories of products including medicines for rare and orphan conditions, and novel antimicrobial medications.

Options which industry opposes or which require significant change

Option 4.1 Approaches to funding or purchasing new health technologies: recognising competition between new health technologies that deliver similar outcomes: 'require offers of a lower price' or 'incentivise offers of a lower price'

Medicines Australia rejects this option. It does not solve any identified issues and it is outside the terms of reference for the Review. Additionally, it contravenes clauses of price certainty in the *Strategic Agreement* and is broadly rejected by industry, patient groups and clinicians as it adds a barrier to entry and reduces patient access. Moreover, this further barrier will have serious negative consequences for patients. It is in patients' best interests to have access to a range of treatment options, ensure diversity of supply and minimise the risk of products leaving the market (or not being launched at all) due to unacceptable post-launch pricing implications. If two medicines confer similar benefit there is no HTA-based reason to justify a lower price. The issue of comparator erosion and lowest cost comparator has been identified as a significant disincentive for new product entry. Compounding the existing poor policy and requiring price reductions below the nadir price of post-patent generic medicines sends a signal to the global market that Australia is not seeking new medicines and that saving money is more important than saving lives.

The proposal of incentivising or requiring Sponsors to propose further discounts is a profound departure from the intent of the HTA review because it will ultimately prevent products coming to

market and lead to unviable price erosion post-launch. There are already many existing price controls including statutory price reductions, reference pricing and post-market reviews amongst other administrative price reductions. We do not support the introduction of new price saving or price reduction measures.

While Medicines Australia supports the consideration in option (2.2) for a streamlined pathway for cost-minimisation submissions to speed up access and improve efficiency, through directing evaluation resources to submissions where they are more needed, we do not support the option to either require or incentivise offers of a lower price.

The impact of this option on stakeholders and the unintended consequences of this policy are discussed in more detail in the following chapters.

Option 3.3 Economic Evaluation: selection of the comparator

This option must be amended to resolve the identified issues. As indicated earlier, the issue of comparator selection and lowest cost comparator has been identified as a significant disincentive for new product entry.

As with other international HTA systems, the principle for the comparator should be 'the therapies most likely to be replaced in clinical practice' [2]. The proposed option to develop guidance and calibrate methods does not move beyond the status quo. The lowest-cost comparator (LCC) issue continues to be of concern to Medicines Australia and the global industry as it links prices of F2 medicines to F1 medicines. Any impact to a new medicine listed in F1 has the potential to flow-on to any other F1 medicines through cost-minimisation and the application of reference pricing. This leads to devaluing of F1 medicines through the HTA process and pricing erosion of F1 medicines over time.

The PBAC and the DOH advise that the legislation (S.101 (3B))[3] prevents them from selecting a therapy likely to be replaced in practice and requires use of the lowest cost comparator. This was confirmed in the expert report by CHERE on HTA Methods: Economic Evaluation. The expert report notes "in practice this means that alternative therapies that **are not the therapy** likely to be replaced **may be relevant** to the assessment for the purposes of pricing". This interpretation of the *National Health Act* (NHA) contributes to delays in access and barriers to entry for future innovative medicines, or no access due to medicines not being listed at all.

RECOMMENDATION: The Reference Committee must make a firm recommendation to Government to resolve lowest cost comparator erosion of value by removing s101(3B) in its entirety commencing before the end of 2024. This would enable selection of comparators to be based on the principle of HTA - "therapies most likely to be replaced in clinical practice" and would allow a weighted price to be derived and accepted during the PBAC recommendation process, where there are multiple

[2] PBAC guidelines, section 1.1.3.

[3] Section 101 (3B) reads as follows:

Without limiting the generality of subsection (3A), where therapy involving the use of a particular drug or medicinal preparation, or a class of drugs and medicinal preparations, is substantially more costly than an alternative therapy or alternative therapies, whether or not involving the use of other drugs or preparations, the Committee:

(a) shall not recommend to the Minister that the drug, preparation or class be made available as pharmaceutical benefits under this Part unless the Committee is satisfied that the first-mentioned therapy, for some patients, provides a significant improvement in efficacy or reduction of toxicity over the alternative therapy or therapies; and

(b) if the Committee does recommend to the Minister that the drug, preparation or class be made available as pharmaceutical benefits under this Part, the Committee shall include in its recommendation a statement that the Committee is satisfied as mentioned in paragraph (a).

therapies which are most likely to be replaced in practice. An alternative approach would be to add a clause to this section as follows:

> Proposed clause *101(6) - 'In situations where there are multiple alternative therapies, Section 101(3B) need not apply*'.

This would enable appropriate pricing and clinical comparators or premiums to be applied to recognise incremental innovation, stay true to the principle that the comparator should be the therapy most likely to be replaced in clinical practice, and enforce the delinking of F1 from F2 as agreed in successive Strategic Agreements.

The impact of the proposed option on stakeholders and the unintended consequences are discussed in the following chapters.

Option 3.3 Economic Evaluation: valuing of long-term benefits

This option must be amended to resolve the identified issue. The proposed option to further consider reduction of the discount rate and conduct more modelling does not move beyond the status quo. As per the *Strategic Agreement* (Clause 5.2), Medicines Australia made a submission to the PBAC in January 2022, calling for the base case discount rate to be reduced from 5% to 1.5%, which would bring it into line with comparable countries that have lowered their discount rates to reflect the increasing value placed on the longer-term future health of their populations. The proposed option further defers the decision for an unknown period. As per the *Strategic Agreement* (Clause 5.2), any reduction in the discount rate was to have been implemented in the PBAC Guidelines by July 2022.

Australia's discount rate must be lowered in line with international best practice and comparable HTA countries to 1.5%, to recognise the value of preventative treatments and cures, and speed up access to these treatments. A reduction in the discount rate need not be offset by changes to other variables such as the incremental cost-effectiveness ratio (ICER), otherwise there will be no net change to the value and hence no recognition of the long-term outcomes and no change in the speed of access. The current discount rate is not consistent with the NMP principle of equity. It disadvantages preventions or treatments given early in life, especially when these result in benefits that extend for the rest of life.

RECOMMENDATION: Notwithstanding the PBAC advice, the PBAC recognises that the discount rate can be lowered as a decision of Government for implementation commencing from 1 July 2024. The Reference Committee must make a firm recommendation to reduce the base case discount rate to appropriately recognise that the value and long-term benefit is accrued over time. Medicines Australia will advocate for a reduction of the discount rate to 1.5% and notes that sensitivity analyses of 0% and 5% can be required in the guidelines.

The impact of the proposed option on stakeholders and the unintended consequences are discussed in the following chapters.

Option 4.1 Approaches to funding or purchasing new health technologies: post-listing reassessment of health technologies

Medicines Australia rejects this option, particularly the framing as an 'explicit disinvestment framework'. There is already a rapid post-market review framework that was updated very recently (February 2024) after a robust consultation process, in accordance with the *Strategic Agreement* (Clause 7.5), and it is not clear what this option adds to the existing process. Framing post-listing reviews in terms of 'an explicit disinvestment framework' presupposes an outcome in favour of the

government for every post-listing reassessment. A more reasonable and symmetrical lens for post-listing reviews would be to frame them in terms of life cycle management rather than disinvestment, and include the possibility that a medicine may become more cost-effective later in its life cycle.

The impact of this option on stakeholders and the unintended consequences are discussed in more detail in the following chapters.

Option 5.6 Strengthen international partnership and work-sharing: collaboration with international jurisdictions to deliver sustainable access to health technologies.

Medicines Australia opposes this option. Rather than speeding up access for Australian patients, such a measure could slow access even further and become a barrier to access occurring at all. As acknowledged in the Options Paper, 'Australia is a small market within a global context'. Australia also has some of the lowest prices in the world compared to similar jurisdictions. If Australia were to join a buying group with other markets, it is expected that manufacturers would need to waive rights to confidential pricing among the payers within the buying group to generate a common price. This would have detrimental international reference pricing implications that would be unviable for manufacturers. It would ultimately result in new health technologies simply not coming to Australia.

In addition, the option refers to "increase market share and purchasing power for innovative health technologies." This is not reflected in the priorities outlined for 2023-24 for the international collaboration, or in its initial set of priorities[4]

Options that need to be amended to meet the intended outcome

Medicines Australia supports the following options in principle but only if they are strengthened as described and only if the details are discussed and negotiated with all stakeholders in a collaborative, co-design approach, with a clear commitment to implementation timeframes and measures of success.

Option 1.4 State and territory government collaboration in HTA: health technologies that are jointly funded by the Commonwealth and State and Territory governments.

Medicines Australia supports a nationally cohesive approach to HTA for technologies that are currently jointly funded, however believes the best way to simplify access pathways for these products is to have them fully federally funded, with all the HTA also done at the federal level. Collaboration with States and Territories will continue to be important for these products.

In order to allow for the holistic consideration of product costs (therapy plus delivery), the States and Territories would need to provide a full articulation of the costs of product delivery, to be included as inputs into the HTA process from the beginning.

Option 2.1 Streamlining and aligning HTA pathways and advisory committees: pathway for drugs for ultra-rare diseases (LSDP)

Medicines Australia supports a streamlined pathway for drugs for ultra-rare diseases, provided it retains the intent of the LSDP to fund these drugs. As such, there should be due consideration that these drugs are unlikely to be cost-effective using the same HTA principles as are applied to other medicines. In the case of ultra-rare diseases with few patients, there is limited data to meet the usual standards of comparative effectiveness and cost-effectiveness expected in HTAs, so a different

[4] PBS News, Sept 2022 and Sept 2023 https://www.pbs.gov.au/info/news/2022/09/collaboration-arrangement-between-the-department-of-health-and-aged-care; https://www.pbs.gov.au/info/news/2023/09/international-hta-collaboration-expands

approach is needed, including consideration of the unique characteristics of the specific diseases and proposed treatment. For example recognition of severe disability/morbidity and/or quality of life will be important considerations for this area . In particular, no comparison can be made between different treatments for different ultra-rare diseases.

The PBAC advisory role to recommend listing of a medicine on the LSDP must be made to the Minister and the CMO as the Minister's delegate as in the existing framework.

Option 2.1 Streamlining and aligning HTA pathways and advisory committees: vaccine pathway

Medicines Australia supports a streamlined pathway for vaccines, provided the ATAGI advice remains sufficiently broad, including advice on the program, clinical evidence and inputs to the economic model, and sufficiently robust. There will be need for transparency in how this advice was considered by the PBAC as per *Option 1.1 Transparency and communication of HTA pathways, processes and decisions*. For the pathway to be effective in speeding up access, *Option 3.3 Economic Evaluation* around selection of the comparator and reduced discount rate must be implemented as per Medicines Australia's proposals above. The proposal for horizon scanning must be linked to the systematic horizon scanning at *Option 5.2 Establishment of horizon scanning programs*. ATAGI should continue to be the authority on the public health perspective of vaccination and should contribute to the recognition of broader components of value that can then be included in the subsequent assessment of value. Streamlining the vaccines pathway must ensure that listing of vaccines is genuinely faster.

Option 2.1 Expanding role of PBAC

While this option clearly refers to the advisory role of the PBAC, Medicines Australia recommends that the option also include reference to the advisory role of the HTA Committee. The term "HTA Committee" is used throughout the document to refer to the single point of assessment. Whether this HTA committee is called the PBAC or something else is not yet clear. The final decision to fund new treatments must remain with the Minister for Health and Aged Care (and Cabinet).

Option 2.2 Proportionate appraisal pathways: triaging submissions

Medicines Australia supports triaging as outlined in the diagram on page 41 of the Options Paper, provided sponsors are able to nominate the triaged pathway. However, there must be clarity for sponsors around the criteria for each pathway, what kind of information needs to be presented for triaging (for example, the PICO scoping step should be at the request of the sponsor to avoid inefficiencies), and what options sponsors have if they do not agree with the decision. Like the other options in this paper, it will be crucial for the triaging process to be co-designed with the industry and other stakeholders

The triaging phase must be appropriately resourced by the Department of Health and Aged Care (DoHAC) so that sponsors can have meaningful interactions with departmental personnel, and not be confined to communicating via the Health Products Portal (HPP), or by emails.

The triaging phase would also be a good opportunity to identify and track HTA applications for orphan drugs through reimbursement pathways so that rare disease specific issues can be identified and addressed.

Option 2.2 Proportionate appraisal pathways: streamlined pathway for cost-minimisation submissions

Medicines Australia supports a streamlined pathway for cost-minimisation submissions but not if it is coupled with option *4.1 'require offers of a lower price' or 'incentivise offers of a lower price'*. We also note that the proposal to share the price of the comparator (against which the proposed

therapy is cost-minimised) with the sponsor early in the process may erode the principle of pricing confidentiality and will not improve listing processes and decisions.

Option 2.2 Proportionate appraisal pathways: early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN

This option lists 3 criteria for the use of early resolution mechanisms.

Criteria a. limits use to "therapies that offer likely HATV in areas where there is HUCN". This criteria may be useful for the pilot stage but does not align properly with the option to expand the resolution step to all relevant cost effectiveness submission.

Medicines Australia recognises that the other two criteria (b. and c.) are about creating an incentive to apply. However there will need to be greater thought given to situations where:

- It may not always be possible to submit TGA and PBAC concurrently – this will depend on the choice and speed of regulatory pathways. Or
- Restricting early resolution to products where the submission is lodged within 6 months of receiving first regulatory approval from FDA/EMA may lock out important medicines where, for example, a global company has acquired a small biotechnology company with the purpose of the expanding the market for these medicines to countries like Australia, but the medicines in question have been registered more than 6 months ago.

Medicines Australia broadly supports alternative option 4*: introducing an optional resolution step **after** HTA committee consideration but **before** advice is finalised*. However, there is much detail that will need to be co-designed in order to ensure this option delivers a speed-of-access advantage compared to the current process. Medicines Australia rejects options 1, 2, and 3 as they add unnecessary restrictions to sponsors and will discourage applications for new medicines in Australia leading to worse access than currently. This directly contradicts the purpose of this review.

In terms of the allowable number of submissions, Medicines Australia considers that any cap on the number of submissions could unfairly deny access for patients. A more appropriate approach might be for the Department and sponsor to discuss criteria for future submissions, so that medicines which benefit patients are not locked out arbitrarily.

There is no detail provided on the 'independent arbitration' process – this should be a feature of option 4.1 *Approaches to funding or purchasing new health technologies: pricing offer and negotiation guidance framework*.

RECOMMENDATION: The Reference Committee should endorse alternative option 4 as the preferred option and seek to have it co-designed with the relevant stakeholders to ensure it delivers a speed-of-access advantage. The Reference Committee should reject a formal cap on the number of submissions, and call for more detail on the independent arbitration process.

Option 2.2 Proportionate appraisal pathways: expanding early resolution step to all relevant cost-effectiveness submissions

Medicines Australia welcomes this option and believes it is critical to expand the early resolution step to all relevant cost-effective submissions after a successful pilot; however, the pilot should be as timely as possible (one or two PBAC cycles) in order to drive faster access for patients. It will be important not to lose the benefits of the current resubmission pathways (such as early re-entry). These must not be disbanded without industry agreement.

Option 2.2 Proportionate appraisal pathways: case manager

For this option to add value, the case manager must have appropriate expertise to engage meaningfully with the submission and sponsor and to provide informed guidance. Interactions should be through direct discussion, not via the HPP or email. As such, a different approach should be taken than the current approach for the facilitated pricing pathway.

Option 3.2 Clinical Evaluation Methods: develop an explicit qualitative value framework

The qualitative value framework is crucial to ensure that Australia's HTA system delivers on society's needs and preferences for medicines.

Non-health benefits such as productivity, caregiver benefits, and equity of access are clearly valued by patients and society. Achieving the goals of faster patient access and Australia as a first-launch country can only be achieved by accounting for a broader and more holistic range of value elements. Explicit, published value frameworks are already recommended and applied by expert committees and organisations such as ISPOR, ICER, NICE and the SMC and Australia must also have a published framework that aligns with international best practice.

Aligned with international best practice, the framework should include explicit consideration of broader value elements including domains not easily quantified in a traditional cost-effectiveness analysis. The value framework must outline the specific value elements the committee considers, how they consider them, and ensure that the impact the value elements have on decision-making are transparent. The background papers explicitly noted that the available academic literature indicates assessments of vaccines often undervalue the importance and societal benefits and that these should be captured as part of the evaluation process. For vaccines, the background papers note published frameworks that include reduced transmission of disease, outcome-related and behaviour-related productivity gains, herd immunity, equity, prevention of AMR and macroeconomic impacts.

The domains must be developed in consultation with a broad range of stakeholders and include elements such as: wider societal benefits (e.g. non-patient outcomes), patient and carer experience (e.g. improvements in convenience and adherence), treatment choice (e.g. alternative mechanism of action or mode of administration), equity (e.g. reduces geographical inequity if hospital admission not required), and real option value (e.g. life-extending treatments may allow for additional treatment options in the future).

The explicit value framework must be incorporated as one of the key considerations within Option 3.3, Economic Evaluation, in particular any consultation on the concept of "Valuing Overall".

RECOMMENDATION: The Reference Committee must make a recommendation to Government that the development of the value framework should be elevated to an independent policy initiative led by a coalition of all relevant stakeholders, and not run by the HTA Committee. This must be done within the first year of HTA reform implementation, so that other elements of reform can be informed by the framework. Once finalised, the value framework should be embedded in legislation to ensure there is no conflict with the *National Health Act*.

Option 4.1 Approaches to funding or purchasing new health technologies: pricing offer and negotiation guidance framework

While it could be useful to codify pricing offers and negotiation, a pricing offer and negotiation framework should only be implemented if it will speed up access, and not add additional steps that

will slow it down. It should be aligned with cost-effectiveness principles and co-designed with industry. It could also feature independent arbitration, as proposed in option in 2.2.

Option 4.1. Bridging funding coverage for earlier access to therapies of likely HTAV and HUCN

The Options paper recommends establishment of bridging funding through a capped special funding program (separate and distinct from the PBS special appropriations) or legislate to enable conditional listings on the PBS.

Medicines Australia supports the capped special funding program **but does not support** legislation that will permit the PBAC to make conditional recommendations. The purpose of the special bridging fund is very clear. Legislating to enable conditional listings goes further and could apply beyond the scope intended for the bridging fund. Similarly for the option on 'approaches for managing uncertainty', Medicines Australia does not support legislative change. Co-development of a workable framework for Managed Access Programs (MAPs) to ensure they are more feasible and implementable would be an appropriate alternative, and could be managed within a Deed.

RECOMMENDATION: The Reference Committee should recommend a special funding program to deliver on the intent of the bridging fund as providing coverage for early access to therapies of likely HATV and HUCN. There must be no legislation change to enable broader conditionality as this would have far-reaching adverse consequences.

Option 4.2 Approaches to incentivise development of products that address antimicrobial resistance (AMR): funding and reimbursement-related changes to support availability of antimicrobials

Implementing payment and incentive models for novel antimicrobials is crucial in the fight against AMR. In proposing a workshop to canvass options, this proposal delays action. There have already been a number of similar workshops in recent years and there are several successful international pilots for subscription models that delink reimbursement from volume that could serve as frameworks for an Australian model.

The Reference Committee should make a firm recommendation to Government to introduce a subscription model as described in the submission from MTPConnect and others. A workshop should be reserved to finalise the details of a delinked model rather than further discuss options.

Option 5.1 Proactively addressing areas of unmet clinical need and gaps in the PBS: early assessment and prioritisation of potentially promising therapies

Any prioritisation around unmet clinical need and gaps in the PBS must not result in other areas being deprioritised (such as rare disease treatments) as this implicitly encourages delays in access. Additionally, there must be full transparency of processes. The New Zealand model of prioritisation is an abject failure, being opaque and not leading to access to medicines for patients. The Committee must explicitly reject the New Zealand model of medicines access and assure the Australian community that access, value and choice remain the cornerstones of the PBS. If implemented, this option should be done in conjunction with horizon scanning.

RECOMMENDATION: The Reference Committee must recommend that any prioritisation must not result in delays in to access for medicines which are not on the priority list (e.g. rare disease treatments).

Option 5.1 Proactively addressing areas of unmet clinical need and gaps in the PBS: proactive submission invitation and incentivisation

Proactive requests for sponsor submissions already occur from time to time. If this is to happen more frequently with the advent of Horizon Scanning, it would be helpful to co-design a framework so that both parties (government and sponsor) know what to expect. The proposed 4-to-6-week timeframe for offer acceptance is not reasonable for a global sponsor to conduct the necessary assessment of feasibility, risks and benefits to the company or find licensing partners if required. Given the uncertainty around the evidence for these medicines (for example for repurposed products), there may need to be incentives for companies to make the proposition viable, for example, through exemptions from the standard price reductions, and exclusivity arrangements.

Option 5.2 Establishment of horizon scanning programs to address specific informational needs within HTA and the health system

Medicines Australia supports the options for horizon scanning and believes all three should be implemented as they are complementary. When considering the implications for the Commonwealth from the introduction of therapeutic advances, it will also be necessary to consider potential legislative changes as well as resources, systems and processes. Importantly, while Medicines Australia believes the recent introduction of an annual horizon scanning forum has been an improvement, to truly make a positive difference and set Australia up for the future, government commitment and resourcing, as well as involvement and collaboration, are required to create an effective horizon scanning process in Australia.

Option 5.3 Consideration of environmental impacts in the HTA: environmental impact reporting

Environmental sustainability is a focus for many medicines sponsors, as well as being a whole-of-government priority. Medicines Australia supports the underlying premise of the need to embed sustainability across all industries. This option would need to be developed in close consultation with the pharmaceutical sector to ensure measures are aligned globally, given the global nature of the industry. It should be a qualitative consideration with a focus on incentivising sponsors to maintain responsible environmental policies, and not penalising them. Additionally, this option should not detract from the fundamental issue of faster patient access.

Option 5.4 Mechanisms for continuous review and improvement

In the interests of continuous review and improvement, it will be crucial to embed agreed key performance indicators (KPIs) in the mechanisms. There needs to be a measure for medicines access that is agreed by all stakeholders, so that progress can be meaningfully measured. This should align with the work being done on performance measures under the *Strategic Agreement* (Appendix 3).

In addition to measuring time to access, Medicines Australia would also recommend specific measures across the dimensions of appropriate value, patient and clinician choice, and investment in innovation. These will be important in understanding whether the HTA reforms which are implemented deliver on the intent of the HTA review.

RECOMMENDATION: The Reference Committee must recommend the early development of KPIs in any implementation timetable so that progress of HTA reforms can be meaningfully measured.

Option 5.6 Strengthen international partnership and work-sharing: work-sharing for individual submissions

Medicines Australia supports international work-sharing for individual submissions if it leads to enhancement of HTA processes and faster access for patients. There is much that HTA agencies can

learn from each other to build capacity and capability across jurisdictions, including education across governments, academics, patients and industry. This option could introduce risks, however, so the following important considerations must first be resolved:

- Have clarity and agreement around the scope and participating markets.
- Prohibit product-specific cost and pricing discussions, given that HTA evaluations address different local health system preferences, variances in patient populations, and different locally available therapeutic comparators.
- Explicitly exclude creation of procurement blocs.
- Continue to protect confidential business information (such as bespoke clinical analyses, cost-effectiveness analyses, pricing information and academic-in-confidence material pending publication) with a guarantee that this information would be safeguarded and not shared across jurisdictions.
- Consider local needs and processes before adoption of international policies.

Options that are not required

Option 2.2 Proportionate appraisal pathways: development of a disease-specific common model for disease areas with high active product development

Medicines Australia opposes this option as it is not required. Disease-specific common models have been trialled in the UK for non-small cell lung cancer (NSCLC) and renal cell carcinoma. It has been reportedly difficult to create a model that captures sufficient complexity to enable it to be used by multiple sponsors where the parameters can be very different respecting different drug classes, disease stages, lines of therapy, dosing regimens, patient populations and so forth. Failure to reflect this complexity in these models will lead to inaccuracies in capturing the full value of the therapy. Rapid changes in disease management and standards of care will also lead to the rapid redundancy of these models. The effort that would be required to develop a range of workable disease-specific common models would likely outweigh any efficiencies gained, and those resources would be better directed to other options where these is more certainty of speeding up access. Australia does not have the capability or capacity to lead in this area.

Q2 What is the potential impact on stakeholders?

For many of the options presented there will be a positive impact on stakeholders through delivering on two of the goals of the HTA Review:

- reducing time to access for Australian patients
- ensuring our assessment processes keep pace with rapid advances in health technologies and scientific progress.

Some of the options, however, will have negative consequences for patients, clinicians and the industry, and will reduce the attractiveness of Australia as a first-launch country, which is one of the terms of reference for the review and a shared goal of the *Strategic Agreement*.

4.1 Approaches to funding or purchasing new health technologies: recognising competition between new health technologies that deliver similar outcomes: 'require offers of a lower price' or 'incentivise offers of a lower price'

This option will have a significant negative impact on Australian patients through increased barriers to entry and reduced access to new and innovative medicines. The details below on the impact to industry help to explain the anticipated negative impact on patients and clinicians.

<u>Impact on the industry</u>

This option has far-reaching implications beyond the discount offered for the individual product due to the government's current reference pricing and lowest cost comparator pricing policies. With this option, each time there is a new market entrant, the prices of products across the whole indication will be reset to the lowest cost comparator in a downward discounting spiral via reference pricing. This would be further compounded by existing pricing policies such as anniversary price reductions and F2 price cuts. This goes against the entire premise of the F1/F2 formulary split, which has delivered significant savings to Government. For F1 medicines, it reintroduces pricing uncertainty (noting that price certainty is a commitment in the *Strategic Agreement* (Clause 7.3)). For F2 medicines, it could reduce the number of competitors and hence diminish the savings from price disclosure and further pressure the supply chain. The way this would happen is described below.

- *Impact on cost-minimisation submissions* (same class or mechanism of action with the existence of a multi-branded comparator linked through therapeutic relativities):
  - A new medicine is referenced to the lowest cost comparator (LCC). This may be in F2 and may already be at the nadir price from multiple rounds of price disclosure.
  - The LCC price is flowed onto existing medicines in F1.
  - The overall value of the therapeutic area is reduced.
  - This creates a disincentive to pursue a cost-minimisation listing.
  - The same consequence would apply for a cost-minimisation analysis with a new mode of action.
  - In some circumstance companies will not launch the medicine in Australia.
  - Other sponsors may withdraw price referenced medicines as the price becomes unviable.
  - Medicines supply chains challenges and medicines shortages are exacerbated.

- *Impact on cost-effectiveness submissions* (new class or mechanism of action with the existence of a multi-branded comparator linked through therapeutic relativities):
    - Through the reference pricing policy, a price reduction in a cost-minimised medicine would flow back to the cost-effective medicine.
    - Over time, this will lead to:
        - Unwillingness of companies to launch in Australia
        - Lower prices for innovative products
        - Pushing Australia down in the launch sequence for innovative medicines
        - Delays and fewer options for Australian patients
        - Less choice for clinician treatment decisions, deterioration of Australia's clinical practice.
        - Withdrawal of products exacerbating supply chain and shortage issues and compromising patients care.

<u>Impact on patients and clinicians</u>

Currently, in many cases, cost-minimisation submissions provide incremental innovation at no additional incremental cost to government, but with benefits to clinicians, patients, and the broader system. This is because many cost-minimisation submissions in Australia are a function of the PBAC's approach to value, whereby patient-relevant benefits (such as improved compliance or tolerability) are of a kind that are not valued, or because clinical trial data and design are unable to quantify these benefits sufficiently to claim superiority. As discussed above, the proposed option of requiring or incentivising lower price offers for cost-minimisation submissions will have the effect of reducing competition, leading to fewer treatment options for patients and clinicians. There will also be longer delays in access as Australia becomes a less attractive country to launch new medicines. Requiring lower prices for subsequent entrants to the market will disincentivise the launch of these products, thereby limiting treatment options and choice, and making Australia less attractive as a first launch country. Further, it may make it harder to attract and conduct clinical trials here, which may in turn further limit the options available to patients. It is well recognised that Australia competes to place clinical trials in Australia, with a competitive advantage in terms of quality of health system, clinicians, and data quality. The work by Professor Ian Chubb, and the Australian Commission on Safety and Quality in Health Care (ACSQHC) is set to improve administration and governance of clinical trials. However, HTA reforms are critical and if HTA and funded access to the market is untenable, none of these other complimentary reforms will achieve their desired impact, patients will miss out and Australia will no longer lead in clinical practice.

3.3 Economic Evaluation: selection of the comparator

The impacts of not having a stronger option for selection of comparators are similar to those discussed above for the cost-minimisation option, because the two issues are very closely linked as described in the previous section. Maintaining the status quo will ultimately disincentivise sponsors from launching early (or at all) in Australia, leading to longer access times, and less choice for patients and clinicians.

(See the recommendation in Chapter Q1.)

3.3 Economic Evaluation: Valuing of long-term benefits

<u>Impact on patients</u>

Australia's discount rate must be lowered in line with international practice to recognise the value of preventative treatments and cures, and to speed up access. If it is left unchanged, it will risk significantly reducing patient access to cutting edge therapies and affecting the long-term future

health of generations of Australians, particularly young people who stand to benefit the most from preventative medicines early in their life. In Australia, the 5% discount rate has contributed to delays in accessing vital therapies, including vaccines for human papilloma virus (HPV) in adolescents, meningococcal disease in children and adolescents, and zoster virus for 60-year-olds and adolescents.

(See the recommendation in Chapter Q1.)

Option 4.1 Approaches to funding or purchasing new health technologies: post-listing reassessment of health technologies

Impact on the industry

The impact of this option on sponsors is significant because it ranges from inappropriate price-lowering power of a monopsony purchaser to involuntary delisting.

Impact on patients

Involuntary delisting's or product withdrawals due to unviable pricing, may lead to patient harms through removal of treatments or changes to alternative therapies (if available). Patients may be required to seek compassionate supply, which may not be offered, or self-fund if products are even available privately.

Q3 Are there any unintended outcomes or challenges stemming from the proposed options?

General challenge: valuing health technologies

The Reference Committee has observed that the primary contentions about HTA economic evaluation methods relate to how health technologies are valued. Many stakeholders who made submissions to the Review assert and describe how the full value of health technologies is not being recognised due to elements of economic evaluations, including comparator selection, base-case perspective of evaluation, discount rate, inadequate approaches to uncertainty, lack of acceptance of non-RCT data, deficient consideration of societal and equity principles, and distributional impacts. Many stakeholders further contend that undervaluing of health technologies delays, and in some circumstances prevents, patients from gaining funded access to advances in healthcare.

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

The 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 appropriately increase the recognised value would proportionately increase the cost of health technologies and require a greater allocation of public resources to fund them.

However, **delayed or denied access to medicines involves far greater costs to society**, health systems and individuals. When considering whether to increase investment in new medicines through consideration of their broader value, the opportunity cost of not making these medicines available as early as possible forms part of the consideration.

General challenge: speeding up access equitably

If the options supported by Medicines Australia are not implemented as a package there is a risk that there will not be faster access across the board, leading to inequities. Medicines Australia supports the creation of a provisional listing fund; however, this will likely benefit only a few listings a year for HUCN. Likewise, a streamlined pathway for cost-minimisation will lead to faster access for those medicines choosing a cost minimised (or cost neutral) approach.

The majority of cost-effective medicines fall outside these two streams and unfortunately the options presented do not offer sufficient opportunities to improve or enable earlier, faster and better access for these submissions.

All Australian patients deserve faster access, no matter which medicines they need. The Government needs to commit to implementing these reforms as a wholistic work program, with all the relevant stakeholders involved in working out the detail.

Option 4.1 Approaches to funding or purchasing new health technologies: recognising competition between new health technologies that deliver similar outcomes: 'require offers of a lower price' or 'incentivise offers of a lower price'

The negative impact of this option on patients and industry has already been discussed in the previous section. A further unintended consequence is that requiring or incentivising offers of a lower price for cost-minimisation will disincentivise companies from bringing products to Australia. It will not deliver on the intended objective of recognising competition. It is likely to have the opposite effect by reducing the number of cost-minimised medicines entering under price reduction conditions, thereby reducing the pool of competition when medicines move into F2. This results in less choice for patients and clinicians, and will also exacerbate the risk of medicines shortages. Ultimately this option will devalue future innovation as it has flow-on implications for drugs listed on a cost-effectiveness basis. It also undermines the shared goal in the *Strategic Agreement* (Recital D) of making Australia a 'global priority for the launch of new and innovative medical treatments', because many global organisations, who require products to be listed at price parity to comparator products, would not be in a position to approve further discounts at the time of launch and may refuse to accept rapid price erosion post-launch as a result of these measures.

A follow-on consequence of Australia not being a global launch priority, is the impact on clinical trials. A company will not consider Australia for clinical trials of a new innovation if there is a risk that the innovation will be undervalued and ultimately not launched in Australia. This impacts access for patients to new therapies especially when they have exhausted existing treatments.

Finally, it is outside the terms of reference of the committee to negotiate price reduction and savings measures. Additionally, it is outside the terms of the *Strategic Agreement* to renegotiate savings measures. The *Reference Committee* must not make recommendations that breach the *Strategic Agreemen*t and fall outside its terms of reference.