Full recommendations of the Health Technology Assessment Policy and Methods Review

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

The Review recommends that the Australian Government:

a. establish a First Nations Advisory Committee, reporting to the Pharmaceutical Benefits Advisory Committee (PBAC) and the Medical Services Advisory Committee (MSAC), to contribute to decision-making across the healthcare continuum, including:
   i. in line with the priority reforms under the National Closing the Gap Agreement 2020, developing a priority list of indications, in partnership with Aboriginal Community Controlled Health Services (ACCHSs), for First Nations people with high unmet clinical need (HUCN)
   ii. developing an active horizon scanning process to identify therapies with promising high added therapeutic value for indications on the priority list. This could include new therapies or new indications for 'repurposing' existing therapies
   iii. advising on proactive submission requests for therapies on the priority list, prioritising those that address areas of unmet clinical need and gaps in access.
b. include representation on the PBAC for First Nations people, including individuals and/or professionals with current expertise and experience in health issues relating to First Nations people, and who are actively engaged in this area. The role should speak to the specific benefits to First Nations people and be accountable for decision-making on behalf of First Nations people
c. require sponsors' submissions to include consideration/assessment of the impact on health outcomes for First Nations people. This will enable more meaningful and informed decision-making by the PBAC and the MSAC
d. develop, in collaboration with ACCHSs, a mechanism to recognise the additional benefit of therapies to First Nations people for the priority health areas listed under the National Agreement on Closing the Gap (2020)
e. develop First Nations HTA criteria. This would support timely listing of medicines with specific health benefits for First Nations people. This could include fit-for-

purpose criteria to allow decisions to be made on a range of population health outcomes, not just one clinical end point

f. resource the Department of Health and Aged Care to assist organisations representing First Nations people to submit and engage in HTA submissions. This would include providing education, support and funding to organisations to develop submissions and be set out in the *Stakeholder Engagement Framework* (See Chapter 6.2).

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

The Review recommends that the Australian Government:

a. formalise a systematic approach for new listings on the Pharmaceutical Benefits Scheme (PBS) to be agnostic of age. This would exclude listings where the Pharmaceutical Benefits Advisory Committee's (PBAC's) reasons for recommending restricted access are based on important safety, effectiveness, cost-effectiveness or quality use of medicines considerations
b. identify, through consultation with stakeholders, existing PBS listings that could be amended to be agnostic of age
c. establish a working party to develop guidance on evidentiary requirements for extending the use of therapies registered with the Therapeutic Goods Administration (TGA) to paediatric populations. The working party should be jointly led by representatives of the TGA and the PBAC and include representation from industry and patient sectors.

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

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

a. Health technology funding and assessment processes, including the level of evaluation, should be fit-for-purpose and proportionate to the level of clinical benefit, clinical need, complexity and financial risk relating to the health technology submission
b. Health technology funding and assessment processes should be streamlined and simplified, with unnecessary complexity removed

c. Consistency and clarity should be created for all health technology submissions for Australian Government funding by developing a unified HTA pathway and committee approach, progressed in stages. This should start with better aligning the HTA pathways, and removing duplication between them. It should be designed to ensure that the health technology funding and assessment processes, pathways and committees support optimal patient care and reduce any fragmentation in care pathways (see 'Recommendation 4. Unified HTA pathway and committee approach for all applications for Australian Government funding for health technologies'). This would be supported by:
    i. the HTA pathway and committee being determined by the assessment requirement for the health technology
    ii. the assessment committee being able to recommend the most appropriate funding mechanism for optimal patient care.

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

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

a. developing a unified HTA advisory committee approach, including changes to the committee composition and scope to ensure it is resourced to consider the breadth of different health technologies. Consideration should be given to the following options for achieving this:
    i. the feasibility of having a smaller core committee membership (for example, 6–10 core members), supplemented by other members drawn from a larger membership pool of different experts, as needed
    ii. the feasibility of having two committees, with the same approach, that have alternating meeting cycles
    iii. the most appropriate submission cycle duration and whether more flexibility or time is desirable and feasible.
b. a staged approach to implementation, with set review points along the stages to ensure changes are meeting objectives. Stages in this process should include the entire or components of:
    i. Recommendation 5. Triaging submissions
    ii. Recommendation 6. Expanding the advisory role of the Pharmaceutical Benefits Advisory Committee beyond the Pharmaceutical Benefits Scheme

iii. Recommendation 11. Proportionate appraisal pathways to align the Australian Technical Advisory Group on Immunisation assessments with the level of risk and complexity of the product
iv. Recommendation 14. Improving time to access life-saving drugs for patients with ultra-rare diseases (Life Saving Drugs Program).

Recommendation 5. Triaging submissions

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

a. submitting all HTA applications for Australian Government reimbursement using a 'single front door' approach.
b. developing a clear and transparent decision tool, such as a decision tree, to guide sponsors' nomination, and to triage selection of the most appropriate HTA pathway
c. establishing a triaging committee for example comprising of the chairs and deputy chairs of the Pharmaceutical Benefits Advisory Committee (PBAC), Medical Services Advisory Committee (MSAC), Australian Technical Advisory Group on Immunisation (ATAGI) and technical sub-committees
d. allowing sponsors to nominate their preferred or appropriate pathway based on their intended submission
e. giving the triaging committee responsibility for considering the submission's product type, risk, complexity, potential benefit and the sponsors nominated pathway to confirm an appropriate and proportionate:
    i. health technology funding and assessment pathway
    ii. HTA advisory committee and technical sub-committees
    iii. Population, Intervention, Comparator, and Outcome (PICO) scoping and engagement approach
    iv. HTA meeting date.

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

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

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

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

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

The Review recommends:

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

for cost-minimisation submissions with the applicant sponsor before the HTA advisory committee considers it.

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

The Review recommends:

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

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

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

Recommendation 9. Therapies with added therapeutic value

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

Recommendation 10. Alternative modelling and analysis types for disease areas

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

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

The feasibility testing should include:

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

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

The Review recommends that the Australian Government:

a. support and resource the development of a framework to assess vaccine submissions for the level of risk and complexity of advice required, building on the advice provided for sponsors through the Australian Technical Advisory Group on Immunisation (ATAGI) Guidelines. This framework should be developed in consultation with stakeholders, including ATAGI, the Pharmaceutical Benefits Advisory Committee (PBAC), and academic experts and evaluators
b. develop a proportionate appraisal mechanism and pathway for vaccine submissions in consultation with ATAGI, the Therapeutic Goods Administration (TGA), the PBAC and industry that:
    i. has a single front door mechanism for vaccine sponsors to make submissions for the National Immunisation Program (NIP) to the TGA, ATAGI and the PBAC
    ii. includes resourcing to enable early triaging and preliminary evaluation of vaccine submissions to determine the complexity and risk of each vaccine submitted (based on the sponsor's self-nomination)
    iii. ensures the level of assessment effort is proportionate to the level of risk and complexity of the submission

iv. is supported by the expansion of the evaluator groups used for the PBAC submissions to include vaccine evaluation experts to assess each sponsor's submission and produce a single comprehensive assessment report (reducing the time needed and duplication, and adding consistency and continuity)
v. aligns ATAGI and PBAC Secretariat processes and functions
vi. includes a process that allows for the ATAGI meeting to occur before the PBAC Economic Sub-Committee (PBAC ESC) meeting to enable the PBAC ESC to consider ATAGI advice.

c. examine the opportunity to delegate to ATAGI the authority to recommend a new vaccine for a disease if the vaccine is being cost-minimised to an existing PBAC-recommended vaccine.

Recommendation 12. Proactive vaccine assessment pathway

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

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

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

The Review recommends that the Australian Government:

a. encourage and provide support for expediting the development and implementation of a nationally cohesive approach to HTAs as outlined in Schedule C of the 2020–25 Addendum to the National Health Reform Agreement (NHRA)
b. develop a national HTA framework, including processes for HTAs to inform advice on implementation, investment and disinvestment opportunities at national and state levels. This work should leverage work already underway through the Health Technology and Genomics Collaboration and align with the unified pathway (see 'Recommendation 4. Unified HTA pathway and committee approach for all Australian government funding of health technologies') where appropriate. The Australian Government should:
    i. develop and implement a methodology, in consultation with stakeholders, to consider the cumulative impact of high-cost, highly specialised therapies (HSTs) on the health system, at a national level. This should be used to inform the HTA decision and reviews of subsidised therapies that ensure the publicly funded therapies being delivered to patients represent the most appropriate treatment pathway for patients
    ii. develop, in consultation with relevant stakeholders, criteria to ensure a nationally consistent process for patient selection and allocation for HSTs.
c. establish time frames for implementing HSTs, funded through the 2020–25 Addendum to the NHRA, where the therapy has a positive HTA recommendation. This should be modelled on targets agreed with respect to the time frames for listing medicines on the Pharmaceutical Benefits Scheme (PBS) (see 'Recommendation 15. Jointly owned performance targets). This process should include:
    i. within 3 months of reaching the in-principle pricing agreement, a national-level implementation plan being published in collaboration with state and territory governments. The plan should include timelines for implementation in the different jurisdictions, and details of how the state and territory governments will ensure their populations (consistent with the HTA recommendation) can access the treatment within 6 months of the in-principle pricing agreement
    ii. an overarching stakeholder explanation of the process following an HTA recommendation, including the reasonable time frames and responsible party for each step (in line with 'Recommendation 3. Overarching recommendations for all health technology assessment funding and assessment pathways and processes'. This information should be developed in consultation with stakeholders

iii. publishing implementation progress information to improve transparency and accountability for all responsible parties (the Australian Government, the sponsor, and state and territory governments), including clearly highlighting causes of delays relative to the developed 'reasonable time frame' for any steps

iv. the original Deed of Agreement to incorporate the requirement for the technology to be reviewed for clinical effectiveness and cost-effectiveness at an agreed period (this will assist with timeliness as it reduces the initial risk, allowing more flexibility).

d. work with state and territory governments and industry to establish (or participate in an existing international collaboration) a horizon scanning process (consistent with the principles for horizon scanning in Chapter 9.2) to identify, prioritise, assess and monitor high-cost HSTs funded through the 2020–25 NHRA Addendum. This process will ensure jurisdictions can begin early implementation planning for HSTs. Additionally:

   i. The horizon scanning should include input from a broad range of stakeholders including patients or patient organisations, industry, academia and the research sector, and state and territory governments

   ii. The horizon scanning should have performance measures to ensure it is efficient and effective for its purpose, and a mechanism for accountability of results to be actioned in preparation for and to inform implementation planning

   iii. Joint funding by the Australian and state and territory governments and industry should be explored.

e. develop a framework for systematic input, consultation and work sharing by state and territory governments across the health technology lifecycle to support efficient and effective implementation and use of health technologies. This includes providing state and territory health departments with opportunities for consultation and collaboration on HTA decisions that will have a significant financial or operational impact on them (see Chapter 9.4).

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

The Review recommends that the Australian Government:

a. develop and publish a statement of rationale for the Life Saving Drugs Program (LSDP) in consultation with stakeholders, including the LSDP Expert Panel. The statement should outline:

i. principles underpinning the program
ii. the eligibility criteria, including the value-for-money consideration, by reference to the overarching recommendations of the LSDP medicines reviews in 2022.

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

Recommendation 15. Jointly owned performance targets

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

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

product, the stage of assessment where progression to listing is primarily delayed should be specified in plain language, as well as the basis for this (where publicly available), using categories such as:

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

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

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

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

The Review recommends that the Australian Government:

a. work with stakeholders (principally industry and government entities) on designing a framework that supports the use of different contract and health technology

funding mechanisms, in addition to the standard 'price per unit' approach. These may include (but need not be limited to) mechanisms that facilitate more timely patient access to high-cost/high-impact health technologies, such as (but not limited to) mortgage-style regular payments, volume-delinked subscription-style reimbursements and/or patient-level product warranties

b. design the framework guided by the principles of:
    i. promoting earlier dialogue, design and negotiation of key parameters and expectations for financing and contracting parameters
    ii. facilitating the adoption of different funding and purchasing mechanisms that address the specific clinical, economic or budgetary issues and uncertainties that may be associated with the health technology in a risk-proportionate manner
    iii. maintaining consistency with other recommendations in this report that relate to pricing, subsidies and risk management–related matters.

Recommendation 17. Pricing offer framework

The Review recommends that the Australian Government:

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

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

Recommendation 18. Updated post-Review framework

The Review recommends that the Australian Government:

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

Recommendation 19. Managed entry agreements

The Review recommends that the Australian Government:

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

Recommendation 20. Bridging funding program

The Review recommends that the Australian Government:

a. establish (after follow-up stakeholder consultations) a bridging funding program to facilitate earlier, temporary subsidised access to promising, time-critical, therapies of high added therapeutic value that address high unmet clinical need (HUCN) for patients
b. design the program in a way that does not introduce unnecessary complexity into the system, nor create unintended consequences that would prolong assessment, negotiations or implementation of agreed terms and conditions between stakeholders

c. in line with international examples, consider establishing a dedicated but separate budgetary allocation for this program, distinct from baseline Australian Government healthcare funding arrangements (e.g. the Pharmaceutical Benefits Scheme (PBS) and Medicare Benefits Schedule (MBS))
d. include in the program design specific eligibility requirements that health technologies must meet to qualify for temporary bridging funding from this program, in consultation with key stakeholders, including:
    i. process-related qualifying requirements – such as earlier submission of health technologies for evaluation via the Therapeutic Goods Administration and HTA parallel submission process – within a defined period after the first major international regulatory approval (e.g. no later than 6 months to 9 months after US Food and Drug Administration or European Medicines Agency marketing authorisation; or within a defined period after identification as part of the HUCN identification process (see Chapter 9.1)
    ii. specific conditions a qualifying health technology must meet and that arose from an HTA; for example, where the HTA advisory committee has recommended:
        1. a cost-effective health technology that requires completion of final negotiations and listings processes
        2. a health technology with outstanding issues (resulting in a negative HTA recommendation due to economic evaluation and/or cost-effectiveness) that can likely be resolved quickly, consistent with facilitated and/or early resolution pathway principles
        3. post-HTA recommendation milestones and conditions, such as:
            (a) (if required) provision of additional information that may address identified uncertainties and risks from the HTA evaluation
            (b) agreement on the costs and duration of bridging funding, to ensure the available appropriation allocations are not exceeded
            (c) clear transition pathways and processes (including stakeholder communications) for the health technology to either:
                (i) transition onto standard subsidy arrangements in the case of a positive HTA recommendation and after agreed conditions are fulfilled, or
                (ii) exit from the bridging program, including details on how residual patient needs for the health technology will be met in the absence of further healthcare payer subsidy, if a final HTA evaluation results in a negative recommendation.

(d) publication (after consultations and necessary redactions) of key terms and conditions for health technologies funded by the bridging program to:
  (i) support visibility of the arrangements and better stakeholder engagement
  (ii) improve patient and clinician participation in addressing specific milestones and conditions.

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

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

The Review recommends that the Australian Government:

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

Recommendation 22. Publishing plain language summaries

The Review recommends that the Australian Government:

a. make plain language summaries of Pharmaceutical Benefits Advisory Committee (PBAC) submissions available at the same time as the PBAC agenda. The summaries would have to be developed in collaboration between sponsors and the Department of Health and Aged Care. Information included should allow consumers (including patient communities and clinicians) to be better equipped to provide input to the HTA process. Additionally, they should provide information for patient communities to understand the expected benefit of the therapy and the proposed population, without ambiguity. Over time, with the earlier engagement of consumers, these summaries may evolve with the consumer along the health technology pathway
b. develop clear, unambiguous and transparent descriptions of committee deliberations that can be understood by patient communities. This includes clear reasoning for recommendations and/or decisions made, and factors affecting decisions (see 'Recommendation 26: Developing an explicit qualitative values framework') including enabling consumers to see how their input was considered and factored into the decision. These should be published where possible or otherwise disseminated broadly to stakeholder groups.

Recommendation 23. Improving the HTA webpage including developing a dashboard

The Review recommends that the Australian Government:

a. enhance access to information about processes, policies and decisions on the HTA website by:
    i. improving navigation
    ii. using accessible language
    iii. tailoring information to specific stakeholder groups where appropriate; for example, clinicians, consumers, health organisations, individual patients and carers, industry and sponsors
    iv. presenting information in a variety of formats, using aids such as case studies and infographics to explain complex topics to stakeholders with differing levels of HTA experience.
b. develop a user-friendly, data-driven, online information platform that makes it easier to find out about HTA processes, outcomes and performance, and includes:
    i. a visual data dashboard for statistics and metrics
    ii. information on individual therapies at each decision point or key milestone
    iii. clear reasons for delays or decisions (including those made by the Government and sponsors) including:

1. standardised reasons for Pharmaceutical Benefits Advisory Committee (PBAC) outcomes for non-recommended therapies (can be multiple reasons)
2. standardised reasons for delays in listing therapies after PBAC recommendations
3. planned implementation timelines for highly specialised therapies (see 'Recommendation 13. Improved processes, accountability and timeliness for highly specialised therapies and other therapies co-funded between the Australian and state and territory governments'*)*, including reasons for any delays on expected time frames.

iv. aggregated information about timelines and decisions, such as for all applications, all medicines claiming additional clinical benefit, therapies for particular indications, and classes of therapies

v. capacity to link information for a therapy across the HTA pathway, including from the Australian Register of Therapeutic Goods (ARTG) application to Pharmaceutical Benefits Scheme (PBS) listing, with consistent standardised recording of indications, populations and drug name

vi. information about when new medicines or expanded indications are first launched globally relative to when they apply for ARTG registration and PBS listing.

c. provide information about the outcome of any proactive submission sought by the Government (see 'Recommendation 46. Proactive pre-HTA processes supporting introduction of identified health technologies for high unmet clinical need').

Recommendation 24. Developing an engagement framework

The Review recommends that the Australian Government develop a stakeholder engagement framework that is guided by the recommendations of the Co-Design of an Enhanced Consumer Engagement Process, this Review, The New Frontier inquiry and *Conversations for Change report*. This framework should describe how and why engagement with stakeholders is used across all HTA processes, from horizon scanning to post-market review. The framework should focus on consumers, including co-design of engagement processes for under-represented communities. Additionally, the framework should acknowledge that the policies, methods and decisions for the HTA pathway have impacts throughout the whole health technology lifecycle and can be used to improve stakeholder engagement outside the direct HTA processes.

Recommendation 25. Improving tinvolvement of consumers in HTAs

The Review recommends that the Australian Government, in addition to other recommendations to improve engagement, inclusion and use of consumer evidence, support consumers to engage with HTA processes through:

a. actively engaging consumers across the HTA system and all relevant processes including horizon scanning; the Population, Intervention, Comparator, and Outcome (PICO) scoping; pre-submissions; evaluations; appraisals; post-market reviews, and disinvestment decisions
b. updating the Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines to specifically request information about how consumers were engaged in the pre-HTA processes including clinical trial design
c. developing education and training to improve consumers' ability to understand and engage with the HTA processes, including how input should be prepared, and how it will be used by committees.

Recommendation 26. Developing an explicit qualitative values framework

The Review recommends that the Australian Government support and resource the development of an explicit qualitative values framework by HTA advisory committees in consultation with a range of stakeholders. The framework should:

a. publish explicit guidance about the elements (beyond clinical effectiveness, cost-effectiveness and financial impact) each committee will consider, how they will consider them, and their impact on decision-making
b. allow enough flexibility for the deliberation process itself to add value to the decisions; that is, not be pre-weighted and scored
c. ensure consideration of the value elements is explicit before, during and after consideration of a technology, and transparently communicate these considerations in public summary documents
d. include documentation on how it will be considered during committee deliberations and guidance, including explaining how sponsors could provide data to respond to additional value elements and explaining how patients and citizens could provide submissions to respond to additional value elements
e. be informed by published research and public consultation
f. include a checklist to assist HTA decision-makers to integrate equity considerations into their deliberations in a more comprehensive, consistent and systematic way. The checklist should account for the fact that some new health

technologies may have a negative impact on health equity. It should also include explicit consideration of priority populations such as First Nations people

g. be consistent with Recommendation 34: Overarching principles for adopting methods in Australian HTA.

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

The Review recommends that the Australian Government develop and implement an Australia-specific framework to optimise timely access to relevant real-world data (RWD) for HTAs, to supplement available randomised controlled trial (RCT) evidence. This framework should:

a. cover enabling systems and pathways, and evaluation and research when collecting and using RWD for HTAs
b. be co-designed and developed with oversight from a multi-disciplinary, multi-stakeholder advisory group, reporting to government. It is important that the group has links to international entities and partnerships with data stewards to facilitate access to data applicable to HTAs
c. include a strategy to increase confidence, awareness and acceptance of cross-jurisdictional and cross-sectoral RWD access and use in HTAs. The strategy should:
    i. centre around consumer and community engagement and co-design, leverage and integrate existing international activities and guidelines, incorporate the Australian context and evidence, and fine-tune responses and messages specific to HTAs
    ii. support the development and enhancement of systems that ensure privacy protections, data security and First Nations data governance, aligning with existing strategies (e.g. the Department of Health and Aged Care's *Data Strategy 2022–2025*).

Recommendation 28. Data infrastructure to support HTAs

a. The Review recommends that the Australian Government develop dynamic, enduring, whole-of-government data infrastructure, with oversight by the multi-stakeholder advisory group (see 'Recommendation 27: Governance and strategic oversight of real-world data to support HTAs'). Consideration should be given to jointly funded infrastructure by the Commonwealth, state and territory governments and industry with potential access to additional stakeholders on a user-pays system.

b. This infrastructure should provide:
    i. transparent and streamlined governance
    ii. the ability to evolve over time, based on the needs of HTA agencies and other stakeholders, evolution of health and digital technologies and assessment tools, and research questions that are likely to be addressed using real-world data (RWD) and real-world evidence
    iii. harmonised international standards, and be flexible and scalable, and allow transparent data quality assessment
    iv. a dedicated consumer-evidence repository to collect data for future HTA activities, track expectations, and support the development of consumer-centred measurement tools
    v. work towards co-designing and building an enduring, sustainable, safe, high-quality and fit-for-purpose data ecosystem that evolves over time to meet Australia's HTA needs.
c. As an immediate priority:
    i. core priority Australian RWD collections that are fit-for-purpose for HTA requirements (including registries and pharmacovigilance data) should be mapped
    ii. access should be facilitated to relevant priority RWD collections for relevant stakeholders to support HTAs
    iii. minimum data quality standards and validation and reporting processes should be implemented for priority RWD collections
    iv. Data should be harmonised across government departments and jurisdictions for government-held Australian RWD collections that are fit-for-purpose for HTAs (including data held by public hospitals and health services)
    v. RWD curation and harmonisation activities should be implemented for priority RWD collections, including internationally standardised coding and terminology.

Recommendation 29. Intergovernmental collaboration in standardised collection and sharing of health technology–related data

The Review recommends that the Australian Government promote state and territory government collaboration and participation in cross-jurisdictional data sharing to support a nationally cohesive HTA system.

This should be facilitated via centralised data-sharing infrastructure and harmonisation of access to existing government-held real-world data collections (see 'Recommendation 28. Data infrastructure to support HTAs').

Consideration should be given to strengthening and supporting the information-sharing clauses recommended in the 2020–25 Addendum to the National Health Reform Agreement, including establishment of information-sharing platform. This would help to ensure that all states and territories meet the policy and resource commitments made in relation to collecting and sharing data on the use of health technologies of interest, from pre-registration to post-market review.

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

The Review recommends that the Australian Government, with oversight by the multi-stakeholder advisory group (see 'Recommendation 27. Governance and strategic oversight of real-world data to support HTAs'), develop a multi-stakeholder coordinated approach to transparent evidence development for HTAs, using best-practice methods, spanning data standardisation, standardised analytics and reporting.

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

The Review recommends that the Australian Government ensure early identification and/or configuration of data collections potentially suitable to help resolve uncertainties (and any new randomised controlled trial evidence), where it is expected that an application is likely to result in a managed entry agreement (MEA).

a. Suitable data collections may include:
    i. priority real-world data (RWD) collections (see 'Recommendation 28. Data infrastructure to support HTAs'), such as in existing clinical registries. Integrated data from a single populous jurisdiction may be fit-for-purpose to address certain research questions)
    ii. in the longer term, outcomes of interest may be collected as add-ons to enduring data linkages, national datasets (see 'Recommendation 28. Data infrastructure to support HTAs') or electronic health records data, as recommended by the advisory group (see 'Recommendation 27: Governance and strategic oversight of real-world data to support HTAs').

b. Where a suitable data collection cannot be identified, establishing new datasets could be considered. These could be obtained via pre-agreed data items (e.g. minimum datasets) collected via relevant electronic medical records or a customised form used for collecting data as a requirement for access to provisionally listed therapies
c. Early exploration and negotiation should begin to determine the feasibility of, and resourcing requirements for, timely, quality data collection and reporting for the intended purpose
    i. Resourcing should be jointly funded by relevant parties, with fund administration and data collection overseen by the multi-stakeholder advisory group (see 'Recommendation 27: Governance and strategic oversight of real-world data to support HTAs')
    ii. Details need to be resolved before entering into any MEA, to provide assurance that uncertainties will likely be resolved via evidence generation during the provisional listing period.
d. Outcomes of interest should be determined based on the areas of uncertainty to be resolved, along with baseline data and information relating to other care received
e. In the case of ultra-rare diseases and other small populations, international collaboration in the collection of patient-level data (e.g. international registries) and the potential use of common data models should be considered, where possible. Inclusion of RWD and real-world evidence obtained through local use should still serve as a mechanism to support funding suitability and clinical effectiveness, as it provides local context.

Recommendation 32. Creating a framework for PICO development to support HTA submissions

The Review recommends that the Australian Government:

a. work with stakeholders to establish framework principles and application criteria that govern when and how the Population, Intervention, Comparator, and Outcome (PICO) framework is to be developed in support of an HTA submission. The framework should:
    i. establish a baseline set of circumstances where a comprehensive and facilitated PICO scoping process would add value to the HTA process
    ii. ensure that any new PICO process facilitates decision-making, reduces the likelihood of resubmission churn, and avoids adding time or complexity to the HTA

iii. ensure criteria of importance to patients and clinicians (e.g. for high added therapeutic value (HATV) that addresses high unmet clinical need (HUCN)) are appropriately considered and discussed as part of PICO development in a manner that:
    1. ensures consideration of relevant patient populations that could potentially benefit from the new therapy
    2. considers the health equity and high unmet need implications associated with access to a health technology
    3. allows discussion of issues that may affect early implementation (for new drugs or major expanded indications claiming added therapeutic value)
    4. captures patient and clinician viewpoints appropriately as part of PICO confirmation outputs
    5. informs the development of the HTA submission and the resulting screening, evaluation and deliberation processes by the relevant HTA advisory committee.

iv. accommodate circumstances where a level of flexibility from the normal PICO scoping and definition process may be appropriate, with clear justification. This may include situations where (for example):
    1. PICO dialogue has been conducted in alternative settings (such as via horizon scanning in stakeholder engagement processes) and information has been collected formally in a manner that serves as an appropriate functional substitute for the purposes of efficient public consultation
    2. a well-defined PICO can be adapted from a comparable overseas HTA entity and has the endorsement of key affected stakeholders to support Australian considerations.

b. establish a process with stakeholders to produce and release plain language summaries of the PICO with the Pharmaceutical Benefits Advisory Committee (PBAC) agenda to:
    i. increase transparency about the proposed treatment population and communicate the expected benefit (outcome)
    ii. assist in managing stakeholder expectations (for new drugs or major expanded indications claiming added therapeutic value).

Recommendation 33. Methods for assessing consumer evidence

The Review recommends that the Australian Government support the development of updates to the Pharmaceutical Benefits Advisory Committee (PBAC) and Medical Services Advisory Committee (MSAC) Guidelines, assessment methods, public summaries and

other explanatory materials to ensure it is clear how both consumer evidence (research into patients' needs, preferences, experiences and perspectives) and consumer input arising from engagement processes (see Chapter 6) may be integrated into HTAs. The updates should:

a. include further guidance on the preparation, use and evaluation of consumer evidence and input, and evidence about equity in submissions. This should include guidance on use of:
    i. qualitative evidence
    ii. patient-reported outcome measures
    iii. patient preference studies
    iv. patient-reported experience measures
    v. evidence about why patients and clinicians in Australia want or need a substitute for current care
    vi. consumer input or use of consumer evidence in R&D relating to the product.
b. clarify how Population, Intervention, Comparator, and Outcome (PICO) scoping; and clinical and economic evaluation steps can be informed by this evidence
c. elicit evidence that would help determine applicability to the Australian setting such as:
    i. evidence from relevant Australian trials (if any), including any evidence specific to Australia in terms of comparative effectiveness and safety and/or patient preferences to inform the clinical evaluation
    ii. evidence from Australian patients about their experiences using the technology, including where patients see its use in Australian practice.

Recommendation 34. Overarching principles for adopting methods in Australian HTAs

The Review recommends that the Australian Government adopt overarching principles for the methods used in HTAs for decision-making about reimbursements. These should include:

a. maintaining preference for:
    i. the best available evidence
    ii. methods that are fit-for-purpose, transparent and only as complex as required to address the problem
    iii. justification of the use of more complex methods.
b. greater acceptance of uncertainty and complex methods where:
    i. managed entry agreements are proposed, and/or

   ii. a health technology is likely to provide high added therapeutic value in areas of high unmet clinical need (HUCN).

c. provision of:
   i. guidance on methodologies preferred by decision-makers
   ii. training and guidance for evaluation groups on new methods
   iii. feedback to sponsors on their use and presentation of analysis based on more complex methods.
d. consultation with stakeholders on adoption of methodologies.

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

The Review recommends that the Australian Government support updates to methods, in consultation with stakeholders, for using non-randomised and observational evidence, in line with the overarching principles for adopting methods in HTAs.

Revised methods should include the following updates:

a. use of indirect comparisons to include the presentation of a comparison of study characteristics, as well as how successful efforts for controlling for differences in characteristics are likely to be
b. creation of control groups to include:
   i. justification of why an indirect comparison is not possible, or less reliable, than the proposed approach of creating a control group
   ii. justification for using methods that are not pre-specified in the study protocol of the proposed technology
   iii. multiple approaches and/or multiple data sources, if possible, and a discussion of any inconsistencies in estimates.
c. use of non-randomised studies to estimate a treatment effect only where:
   i. well justified
   ii. design and analysis elements are sufficiently rigorous to support confidence in decision-making such as through:
      1. prospective design (preferably in collaboration with an HTA or regulatory scientific advice)
      2. registration and transparent reporting
      3. inclusion of multiple sensitivity analyses demonstrating consistency of effect.
d. adjustment of the treatment effect in the presence of treatment switching to include:

   i. multiple methods to be reported to show consistent results. This may include alternative approaches (not only methods to adjust for treatment switching) such as translating intermediate end points unaffected by treatment switching into final outcomes
   ii. a justification of the use of methods that are not pre-specified in the trial protocol of the key study for the proposed technology.

Revised methods should include more guidance on using real-world data (RWD) and real-world evidence (RWE) including:

a. for the data sources that would be acceptable for particular purposes (e.g. costs, utilities and treatment effect)
b. for assessment of the quality of the data source.

Revised methods should ensure RWE is used to determine treatment effectiveness only where the following conditions are met (or there is a strong justification that they cannot be met):

a. the technology is for use in areas of high unmet clinical need (HUCN)
b. higher-quality evidence cannot be generated or will not be generated in a timely fashion
c. limitations of RWE and impact on decision-making confidence are mitigated such as through:
   i. presentation of multiple sources of RWE (including methods of generating RWE from a source, and from multiple RWD sources)
   ii. pre-specification of the use of RWE is in the study protocol for the proposed technology.

Consideration should also be given to setting minimum standards of data quality before data is used in an HTA.

Recommendation 36. Methods for assessing surrogate end points

The Review recommends that the Australian Government support the development of additional methods for using surrogate end points in HTAs, in line with the overarching principles. This guidance should:

a. include circumstances where surrogates would be acceptable (and may include a list of previously accepted surrogate end points paired with use cases)
b. provide further instruction on evaluating evidence using surrogate end points, including methods for identifying the use of surrogates in submissions (as

surrogate relationships can be implicit in economic models but not adequately presented for clinical evaluation).

Development of additional methods should include examination of methods required to validate surrogates to ensure they are consistent with methods used internationally. They should also include methods for describing uncertainty, particularly where surrogate relationships are used in combination with other methods (such as indirect comparisons or model extrapolation) where uncertainty may be substantially increased.

Recommendation 37. Methods preferred by decision-makers

The Review recommends that the Australian Government:

a. support the generation of a curated list of methodologies that are preferred by decision-makers, in collaboration with evaluation groups and sponsors. The list should include methodologies for the appropriate use and assessment of consumer evidence, real-world data (RWD) and real-world evidence (RWE). For each method in the list, brief guidance should be created that includes:
    i. a description of the method including links to key peer-reviewed articles
    ii. guidance for sponsors or evaluation groups on the presentation of the method and results in a submission or assessment report (including a checklist of what data may be required to validate the method) to ensure transparency
    iii. guidance for evaluation groups on how to evaluate the results generated by a method, and how to present uncertainty and the impact of the uncertainty on risk faced by decision-makers
    iv. a brief explanation of how to interpret the results derived by a method
    v. a brief lay explanation of the method for the benefit of patients, clinicians and the broader public to be incorporated into plain language explanation of Pharmaceutical Benefits Advisory Committee (PBAC) and Medical Services Advisory Committee (MSAC) Guidelines.
b. support further training and guidance for evaluation groups when adopting new methods
c. support the provision of feedback to sponsors on their use and presentation of analysis based on more complex methods, and continue this practice for existing pre-submission advice, commentaries, advisory sub-committee and committee advice, or as revised as part of the Review.

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

The Review recommends that the Australian Government:

a. support the development of further guidance on methods for assessing tumour-agnostic therapies informed by:
   i. approaches that have been used by the Pharmaceutical Benefits Advisory Committee (PBAC)
   ii. models proposed in academic literature
   iii. models adopted in other jurisdictions
   iv. consultation with patients, clinicians and industry.
b. support the development of guidance on the assessment and appraisal of genomic technologies and gene therapies for HTA decisions in Australia.

This could be for gene therapies only if PBAC's remit remains as appraising medicines, vaccines, advanced therapies and codependent technologies. Alternatively, if the unified HTA pathway (see 'Recommendation 4. Unified HTA pathway and committee approach for all Australian Government funding of health technologies') is adopted and a single HTA advisory committee is constituted in Australia, it would include companion genomic and pharmacogenomic tests more generally (i.e. for funding decisions for all associated technologies).

As part of the guideline development, a Statement of Principles concerning the access and use of genomic technologies and gene therapies should be co-designed with the public. This would involve stakeholder consultation with patients, clinicians and industry, but also people who do not have an immediate vested interest in these technologies.

Recommendation 39. Discount rate

The Review recommends that the Australian Government:

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

b. determine the base case discount rate for those health technologies as part of its consideration of the financial impacts of implementing the Review's recommendations.
   Note: reducing the discount rate will result in greater attribution of value to future benefits, and may increase the need for performance-based mechanisms that satisfy the HTA advisory committee that uncertainty about future benefits, long-term safety, estimates of cost-effectiveness, and overall cost, will be effectively managed for the period that benefits are claimed.

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

Recommendation 40. Comparator selection

The Review recommends that the Australian Government support updates to the Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines to clarify what alternative therapy should be selected as the main comparator in submissions for health technologies with multiple alternative therapies.

For health technologies that sponsors claim are non-inferior to the selected comparator, updates should make clear that they can cost more than other lower-cost alternatives if the PBAC is satisfied that those lower-cost alternatives:

a. are for compelling clinical reasons, no longer accepted in clinical practice as alternative therapies, or
b. have, for some patients, significantly inferior safety or efficacy.

Updates should provide guidance on the types of evidence the PBAC requires to satisfy itself of these conclusions. This could include:

a. evidence from studies that the health technology provides clinical or other benefits that would significantly improve health outcomes for at least some patients compared with lower-cost alternatives
b. whether the health technology has a different mechanism of action, or other differences, compared to all existing alternatives and there is evidence that significant improvements to health outcomes would be achieved by giving patients and clinicians the choice of multiple different alternative therapies (enabling switching in the event of treatment resistance, failure or intolerance)

c. the extent of use of the lower-cost alternative in contemporary clinical practice, supported by clinical rationale for lower use.

Before updating its Guidelines, the PBAC should seek input from patients, clinicians and industry to identify the types of evidence that sponsors should be instructed may be relevant to the PBAC's consideration. The PBAC should consider the appropriateness of any suggestions before incorporating them into its Guidelines.

Comment by Elizabeth de Somer, Member Nominated by Medicines Australia. 'The industry recognises the importance of the updated PBAC Guidelines that provide clarity to the PBAC and maintains this would be strengthened with an alternative recommendation:

The National Health Act includes an additional clause to clarify that, in subsections 101(3A) and (3B), in having regard to the alternative therapy or therapies for the relevant patient population and any sub-populations, the Committee must consider the therapy or therapies most likely to be replaced in clinical practice.'

Recommendation 41. Cost-minimisation submissions

The Review recommends that the Australian Government investigate mechanisms to differentiate cost-minimisation submissions based on their proportionate benefit and relative cost in line with other options in the Review to calibrate the methods and level of appraisal to the level of risk and clinical need and/or benefit of submissions.

Recommendation 42. Valuing and pricing

The Review recommends that the Australian Government conduct research to understand if and when it may be reasonable for HTA advisory committees to accept higher prices for health technologies than are currently accepted. This includes:

a. in what circumstances
b. for what benefit
c. how much greater cost would be reasonable to secure the benefit
d. the level of confidence needed that the benefit would be secured
e. measures that would be appropriate to offset the higher costs over a product's lifecycle.

To ensure the sentiment captured through workshops and consultations is representative of the Australian population, they should include a population representative sample

(including representatives of key stakeholder groups) and ensure measurement is free from selection bias.

Note: workshops could also be assisted through the use of the explicit qualitative value framework proposed above (see 'Recommendation 26: Developing an explicit qualitative values framework').

Recommendation 43. Environmental impact reporting

The Review recommends that the Australian Government, in line with the *National Health and Climate Strategy*, investigate the following options in consultation with industry and other stakeholders:

a. reporting of environmental impacts, starting with embodied greenhouse gas emissions, during the assessment of cost-effectiveness by Australian HTA bodies
b. potential to use this data in approval and reimbursement decisions
c. potential for public reporting of this data, to inform clinical decision-making
d. the development of guidance documents and examples to facilitate environmental impacts reporting
e. alignment with international best practice in comparable jurisdictions
f. the role of international standards for calculating the carbon footprint of health technology products.

Recommendation 44. Identifying therapeutic areas of high unmet clinical need

The Review recommends that the Australian Government:

a. develop criteria for ongoing identification of therapeutic areas of high unmet clinical need (HUCN) in partnership with clinicians, industry, patients and patient and/or community organisations to:
    i. inform further stakeholder dialogue, horizon scanning and related proactive pre-HTA activities that improve health literacy and health equity
    ii. provide a mechanism to publicly signal the need for, and gauge interest in, making available and subsidising certain health technologies that address specific therapeutic needs in the Australian healthcare context (such as First Nations and paediatric health), but are not available due to limited commercial and operational interest from sponsors
b. in developing and consulting on the criteria, have regard to:

   i. priorities developed through other government activities (such as, but not limited to, outreach activities supporting the Medical Research Future Fund (MRFF))
   ii. antimicrobial resistance surveillance information to identify emerging resistance to available treatments, and surveillance of vaccine preventable diseases
c. develop agreed processes that support regular review of and updates to criteria and therapeutic areas
d. support a subset of the criteria being developed in partnership with Aboriginal and Torres Strait Islander Community Controlled Health Services (ACCHSs) to identify priority areas of HUCN for First Nations people, in line with the priority reforms under the National Agreement on Closing the Gap between all governments and the Coalition of Peaks.

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

The Review recommends that the Australian Government:

a. develop a process consistent with the principles of horizon scanning (see Chapter 9.2) for identifying therapies with the potential to be high added therapeutic value for therapeutic areas of high unmet clinical need (HUCN), including:
   i. new therapies that may not be available in Australia
   ii. existing therapies with initial evidence that they could be repurposed for new indications
   iii. existing therapies with initial evidence that changes to an existing restriction and/or authority may address HUCN and/or significant health inequity
   iv. includes in this process a mechanism for partnering with Aboriginal Community Controlled Health Services (ACCHSs) to ensure First Nations population health outcomes and health equity are appropriately reflected.

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

The Review recommends that the Australian Government:

a. establish processes that facilitate proactive dialogue between stakeholders to support the timely development and lodgement of HTA submissions for health technologies that meet the relevant eligibility criteria. Health technologies discussed would be nominated in consultation with clinician and consumer

stakeholders after being identified through the horizon scanning process and the resulting outputs of the process (see 'Recommendation 44. Identifying therapeutic areas of high unmet clinical need')

b. consult on, develop and apply (as appropriate) incentives that will support the development and lodgement of HTA submissions for these therapies, including (but not limited to):
    i. facilitated Population, Intervention, Comparator, and Outcomes (PICO) scoping and development
    ii. fee waivers
    iii. case management support
    iv. prioritised pathway access (through proposed new proportionate HTA pathways)
    v. potential for access to bridging funding programs (subject to HTA advisory committee recommendation)
    vi. data exclusivity arrangements (where applicable).
c. establish process protocols for sponsors with identified health technologies to:
    i. notify their intention to prepare submissions to the Pharmaceutical Benefits Advisory Committee (PBAC) (and application to the Therapeutic Goods Administration (TGA), if applicable) within a predefined period after official invitation by the Australian Government (to be determined in consultation with stakeholders)
    ii. submit and publish project plans detailing the timing of key milestones supporting the preparation and lodgement of HTA submissions for the health technology(ies)
d. in cases where market incentives do not attract submissions for therapies for high unmet clinical need (HUCN), consult with stakeholders on potential mechanisms to support registration and access.

Recommendation 47. Horizon scanning

The Review recommends that the Australian Government:

a. establish an Australian horizon scanning function that supports the broad principles of:
    i. improving the quality of HTAs, health policy and stakeholder engagement arrangements when considering the implications of new and emerging health technologies

   ii. improving stakeholder awareness and engagement about technologies that may address important healthcare areas (e.g. high unmet clinical need (HUCN), national healthcare priorities and health equity considerations)
   iii. supporting advice that helps healthcare payers with forward planning and setting priorities.
b. work with key stakeholders to ensure that the scope, audience, purpose, governance and outcomes of horizon scanning are appropriately designed so that information can be used to support evidence-based recommendations and advice that support improvements to health technology access and availability for Australian citizens
c. prioritise horizon scanning activities in areas where early attention is most likely to identify major health advances that address health inequities and HUCN and/or have significant health system implications, including (but not limited to):
   i. advanced therapies and health technologies that require collaboration between multiple healthcare payers and providers (including, but not limited to, states and territories)
   ii. health technologies that may support improvements in health equity (including, but not limited to, First Nations health and areas of HUCN) and national health priority areas.
d. provide adequate resourcing to support effective and efficient ongoing operation of the program after open engagement with stakeholders about costs and related contributory implications
e. establish appropriate governance arrangements after consulting stakeholders, and put in place arrangements to:
   i. review the horizon scanning function periodically
   ii. support necessary dialogue with stakeholders to adjust the function's operation over time to ensure it continues to provide efficient and effective outcomes that meet the agreed purpose(s) and scope(s).

Recommendation 48. Mechanisms for continuous review and improvement

The Review recommends that the Australian Government design and establish (in consultation with stakeholders) a program that supports the continuous review and updating of HTA policy and methods in support of the core pillars of the NMP. This program would include:

a. a selection of review topics informed by:

   i. consultation with internal and external stakeholders on areas where systematic concerns have been identified
   ii. contemporary research into international and interjurisdictional best practice
   iii. findings from key performance indicators measurement and reporting arrangements.
b. a transparent schedule of topics for review and review consultation activities, and designated time frames to complete reviews
c. opportunities for all stakeholders to provide input to reviews
d. reporting of review outcomes and (where necessary) recommendations that (if implemented) would improve the operation of the HTA program.

Recommendation 49. HTA evaluation workforce

The Review recommends that the Australian Government:

a. consult broadly to develop programs that enhance the competency and capability of the HTA workforce, including (but not limited to):
   i. sponsored internships between HTA evaluation groups, health departments and industry
   ii. facilitated secondments between HTA evaluation groups, health departments and industry
   iii. international secondments between HTA collaboration countries.
b. discuss with state and territory health departments, opportunities for developing an inter-government evaluation work group to improve capability development and use of HTA capacity as part of achieving nationally consistent HTAs
c. continue progress on inter-agency collaboration and design relating to common HTA evaluation methodology, as part of supporting testing and (prospective) formal introduction of HTA evaluation work-sharing pathways across participating jurisdictions
d. approve reforms to pilot work-sharing pathways for individual health technology submissions that are submitted across jurisdictions with comparable approaches to HTA evaluation. This would reveal the merits of collaborative evaluation for reimbursement-related activities that – if the experience is positive – could be embedded into the HTA framework. Available pathways should include at least one of the following options:
   i. **Work-sharing initiative pathway** – concurrent reimbursement submissions are lodged in multiple jurisdictions and work on dossier modules is split among participating agencies.

ii. **Comparable overseas agency pathway** – finalised HTA evaluations from comparable agencies are provided for review (with redactions for localised pricing information, as strictly necessary).
iii. **Joint expression of interest HTA pathway** – sponsors are invited by HTA agencies to bring forward priority submissions for joint reimbursement evaluation (e.g. specific rare disease treatments or treatments for narrow indications of relevance).
iv. **Hybrid sequential lodgement pathway** – dossiers may not be lodged concurrently, but access to interim evaluations from HTA agencies that are further along in HTA considerations are shared with the agreement of the sponsor, to facilitate expedited local evaluation.

e. update its parallel scientific advice and early dialogue policies to facilitate discussions with industry sponsors, health technology users (principally clinicians and patients) and HTA and regulatory entities earlier than current arrangements (locally or regionally), where a joint HTA evaluation is under consideration.

Recommendation 50. Supporting architecture resourcing

The Review recommends that the Australian Government give careful consideration to the quantity and alignment of resources (financial and personnel) required to effectively implement any agreed recommendations arising from this Review. This includes:

a. appropriations for new activities
b. additional resourcing necessary to reform and/or strengthen existing functions that are essential to support the HTA process and translate HTA recommendations into health technology access for patients. These functions include, but are not limited to:
   i. health communications expertise
   ii. enhanced stakeholder engagement
   iii. commercial negotiation
   iv. HTA evaluation
   v. triaging and case management support.