Australian Government

Australian Government response to the Standing Committee on Health, Aged Care and Sport report:

The New Frontier – Delivering better health for all Australians

Inquiry into approval processes for new drugs and novel medical technologies in Australia

November 2023

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Introduction

The Australian Government thanks the House of Representatives Standing Committee on Health, Aged Care and Sport for its Inquiry into, and Report on, approval processes for new drugs and novel medical technologies in Australia.

The whole-of-government response to the Committee's recommendations has been coordinated by the Department of Health and Aged Care on behalf of the following Commonwealth agencies:

- Australian Commission on Safety and Quality in Health Care (ACSQHC)
- Australian Digital Health Agency
- Department of Industry, Science and Resources
- National Blood Authority (NBA)
- The Treasury.

Information in this document is current as at October 2023.

Information on approval processes for new drugs and novel medical technologies in Australia

Therapeutic goods, including medicines and medical devices, are regulated by the Therapeutic Goods Administration (TGA), which is part of the Department of Health and Aged Care. The TGA is responsible for ensuring that therapeutic goods available for supply in Australia are safe and fit for their intended purpose. Therapeutic goods must generally be entered on the Australian Register of Therapeutic Goods (ARTG) prior to import, export, supply or advertising, unless an exemption applies.

The Australian Government assists Australians in accessing necessary health services and technologies by subsidising the cost of health-related goods and services. A range of different funding arrangements, including public subsidy, are used through the Pharmaceutical Benefits Scheme (PBS), the Medicare Benefits Schedule (MBS), the National Health Reform Agreements (NHRA) with states and territories for public hospital treatments and through private health insurance benefits for medical devices, such as cardiac pacemakers and defibrillators, regulated under the Schedule of the Private Health Insurance (Medical Devices and Human Tissue Products) Rules, also known as the Prescribed List of Medical Devices and Human Tissues Products (the Prescribed List; formerly known as the Prostheses List).

The Department of Health and Aged Care is responsible for Health Technology Assessment (HTA) of health services and technologies to provide policy-makers, funders, health professionals and health consumers with the necessary information to understand the benefits and comparative value of health technologies and procedures. This information is used to inform policy, funding and clinical decisions, and assist with consumer decision-making. HTA involves a range of processes and mechanisms that use scientific evidence to assess the quality, safety, efficacy, effectiveness and cost effectiveness of health services and technologies.

The HTA process plays a vital role in ensuring the Australian Government's objective of delivering a safe, effective and efficient health care system.

Therapeutic Goods regulation

The TGA is responsible for evaluating, assessing and monitoring products that are defined as therapeutic goods. Therapeutic goods generally fall under three main categories:

- Medicines – including prescription, over-the-counter and complementary medicines, such as paracetamol and echinacea
- Biologicals – something made from or containing human cells or tissues, such as human stem cells or skin
- Medical devices – including instruments, implants and appliances, such as pacemakers and sterile bandages, software devices (including those with artificial intelligence (AI)), personalised patient matched devices and *in vitro* diagnostic tests.

The TGA also regulates 'other Therapeutic Goods', which include items such as tampons and disinfectants.

Therapeutic goods are broadly defined in subsection 3(1) of the *Therapeutic Goods Act 1989*[1] (Therapeutic Goods Act) as goods that are represented in any way to be, or that are likely to be taken to be, for therapeutic use, or for use as an ingredient or component in the manufacture of therapeutic goods. 'Therapeutic use' principally relates to use in humans in connection with:

- preventing, diagnosing, curing or alleviating a disease, ailment, defect or injury
- influencing, inhibiting or modifying a physiological process
- testing the susceptibility of persons to a disease or ailment
- influencing, controlling or preventing conception
- testing for pregnancy, or
- the replacement or modification of parts of the anatomy.

The definition of therapeutic goods also includes biologicals and medical devices (as defined in the Therapeutic Goods Act).

Australia has a two-tiered system for the regulation of medicines, including complementary medicines. Higher risk medicines must be registered on the ARTG, which involves individually evaluating the quality, safety and effectiveness of the product. Registered medicines are assessed by the TGA for quality, safety and efficacy. All prescription medicines, most over-the-counter medicines and some complementary medicines are registered. Lower risk medicines containing pre-approved, low-risk ingredients and that make limited claims can be listed on the ARTG on the basis of the applicant making certain certifications about the goods as part of their application.

[1] www.legislation.gov.au/Latest/C2021C00376

The TGA undertakes around 150 significant prescription medicine or biological assessments per year (new prescription medicine or major variation to an existing medicine, e.g. extensions of indication and new routes of administration), with a median assessment timeframe of 190 days over the past four years. Around 20 percent of these assessments were considered in reduced timeframes (priority or comparable overseas reports), and around 15 percent were granted orphan drug designations.

Health Technology Assessment

The Department of Health and Aged Care's Technology Assessment and Access Division is responsible for two program areas that are directly relevant to the Report's recommendations on HTA of medicines: the PBS and the Life Saving Drugs Program (LSDP).

Pharmaceutical Benefits Scheme

The PBS provides Australians with reliable, timely and affordable access to a wide range of medicines. At October 2023, there were 910 different medicines listed on the PBS. In 2022-23, the total PBS expense for the supply of medicines was $17 billion.

The PBS supports Australia's National Medicines Policy[2] objectives, including ensuring equitable, timely, safe and affordable access to a high-quality and reliable supply of medicines. Medicines are assessed by the Pharmaceutical Benefits Advisory Committee (PBAC), an independent, expert statutory body established under the *National Health Act 1953*[3] (National Health Act) to make recommendations and give advice to the Minister about which drugs and medicinal preparations should be subsidised through the PBS, and which vaccines should be listed on the National Immunisation Program (NIP) schedule.

The PBAC considers the effectiveness and cost of a proposed medicine compared to alternative therapies. The PBAC recommends maximum quantities and repeats, and may also specify that a medicine should only be subsidised in certain circumstances.

PBS program requirements, including the functions and role of the PBAC, are prescribed in the National Health Act.

Provisions of the National Health Act and government policies and processes for the PBS are supported by Strategic Agreements with Medicines Australia and the Generic and Biosimilar Medicines Association (GBMA), agreed on 6 September 2021.[4] The agreements include commitments to a range of reforms and reform processes to secure and maintain the best possible access for Australians. This includes reforms to ensure that assessment processes for new drugs and novel medical technologies remain fit for purpose, acknowledging challenges identified through this inquiry such as the rapid pace of innovation, and the increasingly diverse therapies being brought to market, including

[2] www.health.gov.au/resources/publications/national-medicines-policy
[3] www.legislation.gov.au/Latest/C2023C00143
[4] www.pbs.gov.au/info/general/medicines-industry-strategic-agreement

precision medicines and combination therapies. The Strategic Agreement with Medicines Australia commits the Commonwealth to:

- seeking early advice from the PBAC as to whether the base case discount rate outlined in section 3A.1 of the PBAC guidelines aligns with international best practice
- supporting and resourcing an HTA Policy and Methods Review that will involve expert examination of several of the challenges identified through this inquiry, including funding pathways for therapies for rare diseases and new and emerging technologies (including cell and gene therapies, and other precision based medicines)
- continuation of improvements to HTA processes co-designed with the medicines industry and assessment of their effectiveness
- participating in a Medicines Australia convened Horizon Scanning forum to identify major therapeutic advances which may enter the regulatory or reimbursement systems (or both) over the following 18 to 24 months
- implementing a new and enhanced early consumer engagement process to capture consumer and patient voices at an early stage of the HTA process
- working with the medicines industry on options for conditional funding arrangements to complement the priority and provisional pathways used by the TGA.

The Strategic Agreements between the Australian Government and Medicines Australia and the GBMA will be in place for five years from 1 July 2022 to 30 June 2027.

The Australian Government has committed to the implementation of the Strategic Agreements in full.

Life Saving Drugs Program

The LSDP is separate to the PBS and provides approximately 400 patients fully subsidised access to expensive and life-saving medicines for rare and life-threatening medical conditions. Medicines are eligible for the LSDP if they treat life-threatening and ultra-rare conditions (defined as one case per 50,000 people or fewer in the Australian population), and PBAC has found that they are clinically effective, but not cost effective enough to list on the PBS. As at October 2023, there were 18 medicines on the LSDP for the treatment of 11 conditions.

The LSDP Expert Panel assesses applications to include new medicines on the LSDP and provides assistance and advice to the Chief Medical Officer (CMO). The CMO advises the Minister for Health and Aged Care on medicines suitable for listing.

Medical technologies regulation

Many medical technologies are regulated for safety, quality and performance as medical devices. Some technologies fall outside the scope of the therapeutic goods framework, such as household and some personal aids for people with disabilities.

Medical devices generally have a physical or mechanical effect on the body or are used to measure or monitor functions of the body, have therapeutic benefits and are used for humans. This also includes in vitro diagnostic medical devices (IVDs).

There has been an increase in applications received by the TGA for inclusion of medical devices on the ARTG, with an 11 percent increase from 2015-16 to 2020-21. The TGA received 6,713 applications for inclusion of medical devices on the ARTG in 2020-21.

Applications to include a medical device on the ARTG usually rely on evidence of conformity from a comparable overseas regulator. However, the TGA also undertakes conformity assessment certification, which is the detailed evaluation of the manufacturer's quality management system and for high-risk devices also a design examination. Any manufacturer wishing to supply in Australia can seek TGA conformity assessment certification.

Medical and Diagnostic Services and Devices Health Technology Assessment

Public funding of medical and diagnostic services (with or without a device) is considered by the Medical Services Advisory Committee (MSAC), an independent, expert non-statutory committee.

MSAC appraises new medical services proposed for public funding, and provides advice to Government on whether a new medical service should be publicly funded on an assessment of its comparative safety, clinical effectiveness, cost effectiveness, and total cost, using the best available evidence. Amendments and reviews of existing services funded on the MBS or other programs (for example, blood products and blood-related products) are also considered by MSAC.

Sponsors or suppliers of medical devices can also apply for listing on the Prescribed List (formerly the Prostheses List), which sets out the prostheses for which private health insurers must pay benefits (if the patient is covered) and the benefit amount. Prostheses include surgically implantable prostheses and other eligible medical devices, and human tissue items.

The 2020-21 Federal Budget included $22 million over four years to improve the Prescribed List and its arrangements.[5]

Gene Technology Scheme

Some emerging medical technologies involve the administration of genetically modified organisms (GMOs) into patients. GMOs may also be used to synthesise new medicines

[5] www.health.gov.au/health-topics/private-health-insurance/the-prostheses-list/prostheses-list-reforms-and-reviews

or as a diagnostic tool. In Australia, activities with live and viable GMOs are regulated under the National Gene Technology Regulatory Scheme, which is comprised of the *Gene Technology Act 2000*[6] (Gene Technology Act), Gene Technology Regulations 2001 and corresponding state and territory legislation. The object of the Gene Technology Act is to protect the health and safety of people, and to protect the environment, by identifying risks posed by, or as a result of, gene technology, and by managing those risks through regulating 'dealings' (activities) with GMOs.

The Gene Technology Regulator is a statutory office holder who is supported in the performance of their functions by Department of Health and Aged Care staff within the Office of the Gene Technology Regulator (OGTR). Dealings with GMO therapeutics that are regulated under the Gene Technology Act include:

- developing the GMO
- manufacture of the GMO
- use of the GMO in the course of the manufacture of a thing that is not the GMO (e.g. making insulin with a GMO)
- conducting experiments with the GMO (e.g. administering a GMO into a human in a clinical trial)
- import, transportation and disposal of the GMO
- the possession, supply, or use of the GMO in the course of these dealings.

The OGTR also monitors post-market activities with GMO therapeutics. Authorisations for commercial supply of GMOs include ongoing reporting and documentation requirements for licence holders.

While the TGA and the OGTR both regulate the manufacture of a GMO therapeutic, the TGA focuses on making sure the manufactured GMO complies with quality standards and assesses the efficacy and clinical safety of the GMP. In contrast, the OGTR is concerned about the containment of the GMO within a manufacturing facility, to avoid the release of the GMO into the environment, as well as the safety of workers in the facility.

[6] www.legislation.gov.au/Latest/C2016C00792

Australian Government response to recommendations

Recommendation 1

The Committee recommends the Australian Government establish a Centre for Precision Medicine and Rare Diseases within the Department of Health.

- **The objective of the Centre should be to ensure that the capacity of the Department of Health is enhanced to provide Australians with timely access to new drugs and novel medical technologies, including for rare diseases, and that the HTA process and government research agenda aligns with this outcome.**
- **The Centre should provide advice to the Department of Health and the Australian Medical Research Advisory Board on research priorities.**
- **The Centre should provide education and training information including support for patients and a comprehensive horizon scanning unit for new medicines and novel medical technologies.**
- **The Centre should provide advice to governments on the establishment of a dedicated regulatory Health Technology Assessment pathway for cell and gene technologies, in consultation with state and territory governments, industry, patients and other relevant stakeholders. The Centre should regularly provide advice to government on the effectiveness of those pathways and areas for further reform.**

The Australian Government accepts this recommendation in principle and agrees that the Department of Health and Aged Care needs to maintain the capacity to provide Australians with timely access to new drugs and novel medical technologies, including treatments for rare diseases, and cell and gene technologies.

The establishment of a dedicated Centre for Precision Medicine and Rare Diseases requires detailed scoping. The Australian Government accepts the intent of the recommendation, but further consideration needs to be given to the organisational structure to deliver the intent of the Centre.

The Australian Government is already delivering activities that support the intent of the recommendation. The Australian Government is committed to funding quality health and medical research and acknowledges the critical role research plays in contributing to a world-class health system. As part of this commitment, the Medical Research Future Fund (MRFF) provides grants of financial assistance to support health and medical research and improve health outcomes, quality of life and health system sustainability.

Under the 2nd MRFF 10 Year Plan[7] announced in the 2022-23 Budget, $750 million is committed over 10 years under the Clinical Trials Activity Initiative, which provides funding through three streams:

[7] www.health.gov.au/resources/publications/mrff-2nd-10-year-investment-plan

- Rare Cancers, Rare Diseases and Unmet Need (RCRDUN)
- International Clinical Trials Collaborations
- Effective Health Interventions.

The MRFF RCRDUN grant opportunities support clinical trials research that investigates new drugs, devices or treatments for rare cancers/diseases or for areas of unmet medical need.

The MRFF Genomics Health Futures Mission[8] is a landmark investment that will bring a new generation of precision medicine to Australia. The Mission will invest $500 million over 10 years from 2018-19 to 2027-28 in genomics research. It will improve testing and diagnosis for many diseases, help personalise treatment options to better target and improve health outcomes, and reduce unnecessary interventions and health costs.

In addition to the MRFF, the Australian Government is currently exploring opportunities to achieve better research and public health outcomes using data from the My Health Record System. The 2021-22 Federal Budget announcement to enhance the My Health Record system included $9.9 million to implement a research and public health use of My Health Record Data Proof of Concept project over two financial years (2021-22 and 2022-23). The Proof of Concept project aims to establish and test the critical technical infrastructure as well as the end-to-end governance processes and protocols to enable the efficient, secure and ethical sharing or release of My Health Record data for research and public health purposes.

In future, it may be possible to link diverse data sets through national digital health infrastructure, such as My Health Record, to support longitudinal research. For rare diseases, health outcomes can be improved through the ability to look at genomic data alongside medicine and disease data.

Further, a structured approach has been established to enable research opportunities identified within HTA to be developed into competitive grant opportunities funded by the MRFF. $100 million is available over ten years (2022-23 to 2031-32) for this research. The HTA Chairs Committee, including Chairs from MSAC and PBAC, works with the Health and Medical Research Office in the Department of Health and Aged Care to provide advice on the identification, discussion, prioritisation, and refinement of grant opportunities under the MRFF HTA funding stream; supports the monitoring of MRFF grants with relevance to the HTA; and other work as required. This is set out in the MRFF Terms of Reference for the HTA Chairs Committee.[9]

The HTA Consumer Consultative Committee provides strategic advice and support to the principal HTA Committees and the Department of Health and Aged Care. The Committee work plan has included activities specifically relating to consumer

[8] www.health.gov.au/initiatives-and-programs/genomics-health-futures-mission

[9] www.health.gov.au/resources/publications/mrff-health-technology-assessment-hta-chairs-committee-terms-of-reference

engagement and participation in HTA processes. This has included workshops on HTA processes and consumer evidence generation.

The Australian Government has also agreed that Medicines Australia will hold an annual horizon scanning forum with participants in the innovator medicines sector to identify major therapeutic advances which may enter the regulatory or reimbursement systems (or both) over the following 18 to 24 months, and which may represent a significant disruption in the treatment paradigm and/or require innovation in health care system planning. This will allow the Commonwealth to understand potential implications from the introduction of new medical advances in terms of resources, systems and processes, and to inform early engagement of consumers and patients in HTA processes. The first forum was held in December 2022 in Canberra.

In addition, the National Strategic Action Plan for Rare Diseases[10] (Rare Diseases Action Plan) provides guidance and direction around key goals and priorities for Australians living with a rare disease and their families and carers. The Rare Diseases Action Plan highlights three Pillars:

- Awareness and Education
- Care and Support
- Research and Data.

A range of key priorities and areas for action are identified under these three Pillars, including:

- prevention
- early detection and screening
- accurate and timely diagnosis
- access to appropriate care
- supporting people living with a rare disease beyond the health domain
- development of, and access to, relevant therapies
- research and innovation.

Funding of $3.3 million over three years from 2020-21 has been provided for rare disease awareness and education activities; development and delivery of education resources for health professionals; and activities to support people living with a rare disease.

Continued Government focus on precision medicines and rare diseases would align with early findings from consultations undertaken by the Australian Digital Health Agency on behalf of all Australian Governments to refresh the National Digital Health Strategy (NDHS).[11] The NDHS engagements indicate support for federated data sets for

[10] www.health.gov.au/sites/default/files/documents/2020/03/national-strategic-action-plan-for-rare-diseases.pdf

[11] www.digitalhealth.gov.au/about-us/national-digital-health-strategy-and-framework-for-action

treatment and early detection research, overlayed with AI and machine learning to support clinical decision making at the point of care.

In addition, the Australian Government will invest $28.1 million in the period through to 2026 to consider and establish a new national government body to guide the future of genomic health and medicine over the coming decade. This will include consultation and engagement with consumers, researchers, health professionals and industry to determine the core functions and remit of the body. States and territories will also be invited to partner with the Australian Government on this initiative to ensure a nationally cohesive approach to embedding genomics within the healthcare system.

Recommendation 2

The Committee recommends that, consistent with Recommendation 1 and the establishment of a Centre for Precision Medicine and Rare Diseases, the Health Technology Assessment (HTA) process for cell and gene therapies be simplified to establish a clear and certain pathway for such therapies.

- **This simplified process should be considered together with a new HTA pathway for cell and gene therapy.**
- **Building on the Medical Research Future Fund Genomics Health Futures Mission, the Australian Government and state and territory governments should establish a jointly funded national genomics testing program to provide equitable access to genomic testing nationwide. As part of the program, governments should ensure the provision of genomics counselling for all patients.**
- **The Australian Government should prioritise and simplify the regulation of cell and gene therapy pathways for clinical trials in Australia.**

The Australian Government accepts this recommendation in part.

HTA process for cell and gene therapies

A process for the assessment of cell and gene therapies already exists through the current HTA committees arrangements. The commitment to the HTA Policy and Methods Review (HTA Review) in the Strategic Agreement[12] with Medicines Australia includes a commitment to reviewing methods for evaluating new and emerging technologies (including cell and gene therapies, and other precision based medicines) and the suitability of existing funding pathways as required. This is reflected in the terms of reference for the HTA Review, which includes in its scope HTA policy, methods, assessment and funding pathways for Highly Specialised Therapies.

The 2020-2025 Addendum to the NHRA[13] sets out a national approach to the HTA of high cost, highly specialised therapies proposed to be delivered through public hospitals. If an application for public funding is determined to be for a potential Highly Specialised Therapy, MSAC undertakes an HTA on behalf of both the Commonwealth and state and territory governments. Where this occurs, state and territory health representatives are involved across all stages of the process. Once a high cost, highly specialised therapy is recommended for delivery in the public hospital system, the Australian Government makes a 50 percent contribution through the NHRA to the states and territories towards the efficient cost of providing the service. Further, the Australian Government's funding for the first two years of delivery of a new high cost, highly specialised therapy is exempt from the 6.5 percent national growth cap contained in the NHRA.

[12] www.pbs.gov.au/info/general/medicines-industry-strategic-agreement

[13] www.health.gov.au/initiatives-and-programs/2020-25-national-health-reform-agreement-nhra

Genomic testing

In Australia, the joint funding of public hospital services by the Commonwealth and the state and territory governments is governed by the NHRA. Genomic testing is already an in-scope public hospital service provided under the NHRA, and every person with a Medicare card is eligible under the NHRA to receive clinically appropriate public hospital care free of charge. It is unclear what benefit there would be in creating an additional stand-alone program to deliver public services already covered by the NHRA, and this would present a risk of creating further fragmentation in the provision of public health services.

The Australian Government remains committed to supporting genomics testing, including through its investment in the MRFF Genomics Health Futures Mission that will bring a new generation of precision medicine to Australia. The Mission will invest $500 million over 10 years from 2018-19 to 2027-28 in genomics research. It will improve testing and diagnosis for many diseases, help personalise treatment options to better target and improve health outcomes, and reduce unnecessary interventions and health costs.

Cell and gene therapy pathways for clinical trials

In mid-2021, the TGA commissioned a review of Australia's regulatory framework for gene, cell and tissues therapies to provide suggested improvements including for clinical trial pathways. This review and TGA's response have now been published.[14]

While the Clinical Trial Notification scheme is widely acknowledged as a fast and straightforward access pathway, the TGA will improve and provide transparency of timeframes for Clinical Trial Approval scheme applications and provide more accessible communication materials on regulation of clinical trials and an advice service to assist research and clinical organisations to support these reforms.

A single point of entry for all approvals/notifications required to conduct clinical trials in Australia will also be implemented, with work already underway.

[14] www.tga.gov.au/report-cell-gene-and-tissue-regulatory-framework-australia-stakeholder-perspectives-tga-response

Recommendation 3

The Committee recommends the Australian Government establish an Office of Clinical Evaluation within the Department of Health to assess the best and most effective care for patients in the context of new and emerging health technologies.

- **The Office should enable evaluation of both pharmacological and non-pharmacological interventions, combination products and products with different sponsors. It should also establish a "living evidence" function to ensure Health Technology Assessment is based on the most up-to-date global health practices.**
- **The Office, in consultation with relevant stakeholders, should conduct a review of how the Department's Health Technology Assessment system assesses combination products, particularly combinations with different sponsors, with a focus on:**
    - **Value attribution between the different products**
    - **Challenges to cooperation between sponsors due to competition law**
    - **Disincentives for a sponsor with an already listed product to participate in its combination listing**
- **The Office should consider collaboration with the National Institute for Health and Care Excellence (NICE) in the United Kingdom to establish similar clinical evaluation processes in Australia that links in with Australian Health Technology Assessment processes.**
- **The Office should cooperate and share information with the state and territory governments to ensure that patients receive treatment where it is safest and most efficacious for them and that there are no gaps in continuity of care.**

The Australian Government accepts this recommendation in principle, and agrees that clinical evaluation within the Department of Health and Aged Care must continue to support patient access to new and emerging health technologies.

The Terms of Reference for the HTA Review[15] state that one of the objectives of the HTA Review is to present a comprehensive set of recommendations for reforms to Government that ensure HTA policy and methods are well adapted to, and capable of, assessing new technologies that are emerging or are expected to emerge in the coming years.

The HTA Review will consider HTA policy and methods for managing clinical, economic, financial and other uncertainty throughout the lifecycle of a technology including better capture of necessary data on duration of effectiveness and safety events. To support this work, expert groups have been engaged to undertake an analysis of approaches used in clinical evaluation and managing uncertainty internationally. This analysis will

[15] www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-terms-of-reference

include the approaches used by the United Kingdom's National Institute for Health and Care Excellence (NICE) and several other comparable countries.

The terms of reference for the HTA Review also state that it will consider HTA policy and methods for all medicines and vaccines and other health technologies that improve health outcomes associated with medicines and vaccines. This will include consideration of combination products and any barriers to equitable and the earliest possible access. The Australian Government expects that the Reference Committee will consider the input and recommendations arising from this inquiry where appropriate.

The Minister for Health and Aged Care has approved for the Department of Health and Aged Care, together with the PBAC and MSAC, to enter into a collaboration arrangement with five like-minded HTA agencies in the United Kingdom and Canada. Additional Canadian and New Zealand HTA agencies joined the collaboration in September 2023.[16] NICE is a key participant in this collaboration arrangement. The arrangement creates opportunities to explore collaboration and support for shared strategic objectives and priority areas. The collaboration arrangement respects each participant's governance arrangements and independence and does not impose legally binding commitments.

The Department of Health and Aged Care also has a Memorandum of Understanding with Singapore to enhance collaboration between our two countries on HTA.

The Department of Health and Aged Care also collaborates with state and territory governments through the HTA of high cost Highly Specialised Therapies. The 2020-25 Addendum to NHRA sets out a national approach to the HTA of therapies proposed to be delivered through public hospitals. The MSAC appraises Highly Specialised Therapies on behalf of both the Commonwealth and state and territory governments. Where this occurs, all information is shared with state and territory health representatives, and jurisdictions are included across all stages of the process.

The Commonwealth, states and territories will also continue to advance broader HTA reform under the NHRA, which contains commitments to support nationally cohesive HTA reform. The goal of nationally cohesive HTA reform is to increase the consistency, timeliness, efficiency and value of HTA processes in Australia. Intended outcomes of this reform include effective sharing of information and recommendations between jurisdictions and the Commonwealth, including consistent outputs and standardised advice, improved patient access to cost effective health technologies, and coordinated and timely responses to rapidly changing technologies.

[16] www.pbs.gov.au/info/news/2023/09/international-hta-collaboration-expands

Recommendation 4

The Committee recommends that the assessment process for the Life Saving Drugs Program (LSDP) be streamlined and delays in access to treatments be reduced by ensuring that a sponsor only need lodge one application for one Health Technology Assessment pathway. The Committee recommends either:

- **Providing sponsors with an immediate pathway to the LSDP Expert Panel (instead of waiting for a PBAC determination), or**
- **Providing a pathway by adjusting the Pharmaceutical Benefits Scheme section 100 program, with specific criteria, as with other section 100 programs.**

The Committee believes it is critical that consideration be given to how the LSDP will integrate with an increasing number of precision medicine applications into the future.

The Australian Government accepts this recommendation in principle, in part, and is committed to providing more timely access to new medicines for life-threatening and ultra-rare conditions. The commitment to the HTA Review in the Strategic Agreement with Medicines Australia includes a commitment to the review addressing methods for evaluating medicines for rare diseases for PBS reimbursement and alternative funding pathways. This is included in the terms of reference for the HTA Review.

Sponsors may already apply for PBS listing for medicines for ultra-rare diseases through a single application to the PBAC. However, where sponsors wish to seek listing on the LSDP because they do not agree to provide the medicine at a comparatively cost effective price (as required under legislation for listing on the PBS), then a further application to the LSDP Expert Panel is required.

As a statutory body, the PBAC can only perform functions set out in its enabling legislation, the National Health Act. Amending the National Health Act to give the PBAC the function of advising Government on LSDP listings is one potential pathway to address this recommendation. Adjusting PBS section 100 listing requirements may also require legislative amendment. Policy and legislative options will be explored as to how this recommendation can be implemented, while ensuring the long-term sustainability of the PBS.

The alternative recommendation to set up a medicine listing pathway that bypasses the PBAC is not accepted. The Australian Government affirms that the PBAC remains the pre-eminent source of advice to Government on the clinical effectiveness and cost effectiveness of new medicines.

Recommendation 5

The Committee recommends that the Australian Government develop a labour market and skills strategy to expand the number of health economists in Australia. This could include encouraging training within Australia as well as seeking expertise from overseas.

The Australian Government accepts this recommendation, recognising the need to expand the number of health economists within Australia to fully support the Department of Health and Aged Care's HTA functions.

A labour market and skills strategy will allow the Australian Government to rigorously assess the strengths and weaknesses of the domestic market for health economists, based on a strong evidence base. A formal strategy will help Government make more informed policy decisions to improve the labour market for health economists. It will also help bring greater consistency and coordination to the issue.

The Australian Government will explore a range of options to achieve this outcome.

Recommendation 6

The Committee recommends that the Department of Health increase its efforts to educate and engage with patients, clinicians, industry and the public and develop education campaigns on all aspects of the regulation and reimbursement system.

The Committee recommends that the Department of Health improve information available on the websites of the Therapeutic Goods Administration (TGA) and its Health Technology Assessment (HTA) bodies for all users including patients, clinicians, industry and the public. This would include:

- **Using plain English language, infographics and videos to explain general processes and timelines**
- **Explanations on the TGA and all HTA websites of how that entity fits into the overall regulation and reimbursement system, similar to the Medical Services Advisory Committee's Australian Government HTA Processes factsheet.**
- **The Department of Health expanding the Pharmaceutical Benefits Scheme Medicines Status website to include technologies funded through the Medicare Benefits Schedule or create an equivalent website for such technologies.**

The Australian Government accepts this recommendation.

Public education on regulation and reimbursement

The Australian Government accepts this part of the recommendation. The Department of Health and Aged Care currently conducts a range of educational and engagement activities with stakeholders and regular meetings with industry on aspects of the Australian regulation and reimbursement system. This includes presenting at medical and consumer conferences and providing webinars and workshops as well as written material. These activities have increased over the last two years, due to increasing online delivery capability built during the pandemic, and will continue to be developed and expanded, leveraging off updated information for the Australian HTA webpages.

Further, several fora are used to facilitate consultation with the medicines industry, with the primary forum being the Access to Medicines Working Group (AMWG). The AMWG is a collaborative committee comprising representatives of the Department of Health and Aged Care, and Medicines Australia. The Minister for Health and Aged Care has oversight of the AMWG and its policy priorities. The AMWG also has a range of subgroups, each of which focuses on a range of specific issues, including cost recovery arrangements and the transparency and efficiency of listing processes.

In addition, the TGA is continually striving to improve its communication and education of all aspects of therapeutic goods regulation for a diverse range of audiences including patients, clinicians, industry and the public. The TGA is informed by its Consumer Engagement Strategy that sets out principles designed to positively influence consumer health by:

- providing quality and tailored information through various channels
- driving service improvement through feedback from the end users of health products
- designing solutions that meet consumers' needs and empowers them to have informed decisions with health professionals.

As part of the Budget 2023-24, the TGA will receive funding for four years for activities aimed at protecting public health ($61 million over four years), which includes (but is not limited to) increased engagement and information to consumers and health professionals and providing continued assistance and education to small and medium enterprises particularly those developing emerging technologies

Website improvements

The Australian Government accepts this part of the recommendation and work is already underway to update website content and structure. Content on the Australian HTA webpages is currently undergoing a significant review, with improvements implemented progressively during 2022. The websites now include new content that better describes HTA processes in more accessible language. Improvements are continuing and may include infographics and resources aimed to assist stakeholders in understanding the regulation and reimbursement system.

Likewise, the TGA is developing information and education resources that use plain English and engaging formats (such as infographics and videos) delivered through channels that are accessed by the target audiences to maximise the opportunity for consumer awareness. It is important to note, however, that TGA's industry cost recovery model currently limits a very significant extension of consumer outreach activities.

As part of the Digital Transformation Strategy announced in December 2019, the complete redevelopment of the TGA website is underway following extensive feedback from consumers, health professionals and industry. The TGA website redevelopment includes a content audit and rewrite of content in plain English, improved information architecture to assist in finding information, support to improve user experience when making regulatory submissions, and work on digital self-service options.

Expansion of Medicines Status Website

The Australian Government accepts this aspect of the recommendation.

The Medicine Status Website[17] was developed to enable consumers to search for and monitor the status of medicines as they progress through the PBS listing process.

For services being considered by the MSAC, webpages are already available for each technology outlining the progress of the application through the MSAC process and stakeholders are able to search using keywords for applications.[18] Over the last

[17] www.pbs.gov.au/medicinestatus/home.html

[18] Accessible from www.msac.gov.au/internet/msac/publishing.nsf/Content/application-page

12 months, improvements have been made, and continue to be made, to the MSAC webpages. The Department of Health and Aged Care is considering how best to capture timeframes information, including whether such information would best be displayed on the MSAC webpages, included as part of the Health Products Portal, or provided on a separate resource similar to the Medicines Status Website.

Building the capacity of consumers

The Strategic Agreement with Medicines Australia includes a commitment to implement an enhanced consumer engagement process to capture consumer and patient voices in respect of applications to list new medicines on the PBS. Through this work, the Department of Health and Aged Care will continue to engage with consumers to understand what information is needed to ensure the patient voice is heard.

The HTA Consumer Consultative Committee was established in 2017, and the Consumer Evidence and Engagement Unit (CEEU) was established in 2019 to support broader consumer participation strategies across HTA. The CEEU has hosted a range of workshops and meetings with consumer organisations aimed at better supporting consumer representatives to engage effectively with HTA processes, including Consumer Symposiums aimed at improving engagement and communication with patients and consumer groups on HTA-related matters. Similar activities are planned across 2023 and will include strategies looking at how best to build the confidence and capacity of consumers and consumer organisations to contribute effectively to HTA processes.

Recommendation 7

The Committee recommends that the Department of Health and the National Blood Authority, in consultation with state and territory governments, reform the Health Technology Assessment processes for blood products to provide better alignment with the Health Technology Assessment system, including:

- **Publication of guidance documents for applicants**
- **Establishment of timelines for applications, and publication of an assessment cycle calendar**
- **Creation of a parallel Therapeutic Goods Administration and Health Technology Assessment process.**

The Australian Government accepts this recommendation, noting there are unique features of fresh blood and other blood products that will need to be factored into any review of current assessment processes. For example, in addition to considering the level and type of evidence that is appropriate for rare disease therapies, treatments funded under the national blood supply require the consideration of Commonwealth, state and territory governments due to the joint funding arrangements. Further review of current HTA processes for blood products, including the development of guidance and potential assessment timeframes, will also need to be considered by governments.

The NBA is preparing a guidance document so that applicants can more easily understand the application process, which will be done in consultation with stakeholders. It will also be informed by the submissions to this inquiry, the requirements of the National Blood Agreement, and the practical operation of assessment processes.

The Department of Health and Aged Care, including the TGA, and NBA are already working collaboratively to investigate efficiencies that might be gained through the creation of further parallel HTA procedures. This would build on existing informal interactions and information sharing between the Department of Health and Aged Care, TGA and the NBA to support HTA processes.

Recommendation 8

The Committee recommends that the Australian Government make the following changes to submission fees for the Therapeutic Goods Administration (TGA) and the Pharmaceutical Benefits Advisory Committee (PBAC) and where appropriate Medical Services Advisory Committee (MSAC) assessments in the following separate circumstances:

- **Replace the current orphan drug fee waivers with a HECS-style fee waiver, in which orphan drug application fees are payable on successful application, only once the drug has earned the sponsor a certain amount of revenue. The Department of Health should determine this threshold value in consultation with industry.**
- **To support smaller companies, HECS-style fee waivers for any sponsor company with revenue at or below $50 million per annum.**
- **HECS-style fee waivers for Australian start-up companies with a specified amount of revenue in the Australian market to promote innovation.**

The Committee also recommends introducing a sliding scale for fees for resubmissions, with fees being lower for resubmissions.

The Australian Government notes this recommendation.

A Higher Education Contribution Scheme (HECS)-style fee waiver would involve interest-free loans with repayment starting when taxable revenue reaches a certain repayment threshold. Outstanding loan amounts would be indexed annually in line with the Consumer Price Index to maintain real value of debt.

The Australian Government remains committed to ensuring safe and clinically effective new medicines and health services are available to Australians who need them. The Australian Government will continue to consider the potential for both fee arrangements and waivers (including HECS-style waivers) that support smaller companies when introducing cost recovery arrangements for HTA processes.

TGA submission fees

Implementation of the proposed HECS-style fee waiver model would require detailed consideration by Government of how it could be funded, as submission assessment costs for non-orphan drugs are currently fully cost recovered irrespective of the projected revenue from sales of the product. Under the proposed model, for each orphan drug considered, the TGA would incur the full TGA salary and other costs of each evaluation up front, but depending on revenue from sales of the product would either recoup those costs some years later, or not at all. Monitoring sales would also incur audit costs. Any differential based on revenue of the sponsor company rather than the product itself not being financially viable to market in Australia (which is currently part of the criteria for orphan drug eligibility) may be seen to reflect the cost of investing in research and development of orphan products and reduce overall incentives for development and application.

The current orphan drug fee waiver mechanism aims to a strike a balance between subsidising orphan drugs, while not providing incentive for submissions to be either premature or of poorer quality.

PBAC and MSAC submission fees

The Department of Health and Aged Care conducted a public consultation process in January 2022 regarding the development of a cost recovery proposal for MSAC applications including the feasibility of a HECS-style fee waiver. Through this process, stakeholders have identified a number of complex policy options which the Department is working to explore in detail, and will consider incorporating into an amended proposal.

The development of a cost recovery proposal for MSAC applications was supported by a Portfolio Charging Review undertaken by the Department of Health and Aged Care in conjunction with the Department of Finance in 2022.

The Department of Health and Aged Care currently has a fee waiver mechanism in place for PBAC submissions and is actively considering the inclusion of waiver and exemption mechanisms in future MSAC charging models. This mechanism allows a waiver to be granted if the PBAC submission is of high public interest and payment of fees would make the submission financially unviable to the applicant. Exemptions to fees are also available for a variety of reasons including due to public health events or temporary supply issues.

Other issues

A move to introduce fee waivers for Australian startup companies would need to take into account the views of Australia's international trading partners. Few regulators have differential fees for review of applications from local and international companies.

While the Australian Government notes the intent behind the fee sliding scale for resubmissions, it is inconsistent with the requirements of the Australian Government Charging Framework.[19] This Framework requires Departments of State to recover the efficient cost of activities generated by stakeholders.

[19] www.finance.gov.au/government/managing-commonwealth-resources/implementing-charging-framework-rmg-302/australian-government-charging-policy#australian-government-charging-framework

Recommendation 9

The Committee recommends that the Australian Government establish a fund to support patients, clinicians and non-profit organisations to sponsor registration and reimbursement applications where there is no realistic prospect of a company serving as sponsor, and where the Department of Health is otherwise supportive of the application.

- **Such a fund should be targeted at treatments for conditions where low patient numbers in Australia serve as a market barrier and where there is a clinical demand and need. The fund should be available for applications to repurpose previously listed medicines and technologies.**
- **The fund should be annually capped with clear and transparent eligibility rules.**

The Australian Government notes this recommendation.

The Department of Health and Aged Care currently has several mechanisms in place to financially support applicants seeking registration of health technologies via the TGA, subsidy through the PBS, NIP or the LSDP, or public funding through the MBS or other Commonwealth funding programs.

There is currently no legal or procedural barrier to non-profit organisations serving as a sponsor. However, there may be practical barriers to non-profit organisations acting as sponsors for products such as repurposed medicines. Any sponsor needs to be able to undertake full product stewardship roles throughout the product life cycle, including safety monitoring and reporting (pharmacovigilance) and ensuring manufacturing quality, which remain essential for public safety reasons.

Medical conditions where there are low patient numbers (less than 5 in 10,000 people) are considered to be rare diseases. The Department of Health and Aged Care is currently working to address rare diseases at a national level via the Rare Diseases Action Plan.[20] There are also several programs and initiatives that support the treatment and management of rare diseases including the TGA's orphan drug program and PBAC cost recovery policy.

The TGA's orphan drug program waives application fees for applications seeking public funding for new medicines to treat rare diseases with low patient numbers and where payment of fees would make the medicine financially unviable to market in Australia. The program also includes a pathway to seek orphan designation for new dosage form medicines. This pathway is intended to provide support to register medicines on the ARTG that would not be financially viable in the absence of a TGA fee waiver.

For applications seeking PBS or NIP listing, there are two mechanisms in place to financially support applicants. Applicants have the option to apply for fee exemptions or fee waivers for submissions that meet certain criteria. Fee exemptions are applicable for submissions in relation to TGA designated orphan drugs for treatment of rare diseases

[20] www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases

with low patient numbers. Fee waivers are applicable for submissions that involve the public interest and for which payment of fees would make the submission financially unviable.

As at October 2023, the LSDP includes 18 life-saving, high cost medicines that are not listed on the PBS for 11 ultra-rare conditions, providing physical and financial relief for approximately 400 eligible patients annually. There is currently no additional cost to sponsors in bringing forward applications to the LSDP. There is also no cost to eligible patients in accessing these very high cost medicines and no cap on funding available for the program.

For applications to MSAC seeking public funding for new medical services to be listed on the MBS or other Commonwealth programs, applications can be (and are) made to the Department of Health and Aged Care by the medical profession, medical industry and others, including clinicians and non-profit organisations with an interest in seeking Australian Government funding for a new medical service, health technology or change to an existing service. The applicant has the option to request a Department-funded HTA, including for applications related to medical services and health technologies for treatment of conditions with low patient numbers. A Department-funded HTA includes a Population, Intervention, Comparator and Outcome (PICO) assessment and also a Departmental Contracted Assessment Report. For future MSAC charging models the Department of Health and Aged Care is actively considering the inclusion of waiver and exemption mechanisms.

Recommendation 10

The Committee recommends that the Australian Government amend the *National Health Act 1953* (Cth) to give the Pharmaceutical Benefits Advisory Committee the power to authorise Managed Access Programs. The eligibility criteria for these Managed Accessed Programs should be aligned as far as possible with the eligibility criteria for the Therapeutic Goods Administration's provisional registration.

The Australian Government accepts this recommendation in principle, noting that amendments to the National Health Act can only be made by the Australian Parliament.

The Managed Access Program is an existing mechanism that enables the listing of medicines registered by the TGA on the PBS under special circumstances of high unmet clinical need, on terms that allow for the resolution of otherwise unacceptable clinical or economic uncertainty for the PBAC. The PBAC is able to recommend products for listing under a Managed Access Program as outlined in the 2015 framework.[21]

While these arrangements can lawfully be given effect under current legislation, options to specifically legislate the arrangements will need to be considered by the Australian Government.

Under the Strategic Agreement with Medicines Australia, the Commonwealth has acknowledged the need to complement the priority and provisional medicines pathways used by the TGA, and agreed to work with Medicines Australia to consider options for conditional funding arrangements. Matters in respect of efficient and equitable funding approaches and pathways for medicines will also be considered as part of the HTA Review.

[21] See www.pbs.gov.au/industry/listing/elements/pbac-meetings/pbac-outcomes/2015-03/Overview-of-managed-access-program.pdf

Recommendation 11

The Committee recommends that the Department of Health conduct a comprehensive consultation process with industry to establish a more flexible way forward for the repurposing of drugs in Australia. This should include:

- **Establishing a new pathway that incentivises the repurposing of drugs for all diseases, not just rare disease.**

The Australian Government accepts this recommendation.

The Australian Government through the Department of Health and Aged Care is introducing a medicines repurposing program to improve patient access to treatments by assisting sponsors to expand the approved uses of their medicines. The program was announced as part of a broader PBS reforms package in the 2023-2024 Budget[22] and is expected to open in early 2024.

The program will target medicines for which a significant public health benefit has been identified but there is little or no commercial incentive for a sponsor to pursue regulatory approval and PBS listing to make this use more accessible. The program will strengthen the voice of clinician, academic and patient groups by encouraging their active participation in identifying new usages of medicines.

The Department of Health and Aged Care commenced work on the medicines repurposing program in early 2021 and has undertaken an extensive consultative process with stakeholders in the form of workshops, roundtables, and public consultations during 2021 and 2022.

The program will support and provide incentives for sponsors through the regulatory processes for the TGA and reimbursement processes for the PBS with the aim of adding repurposed medicine usages as cheaper alternatives for health professionals to prescribe for consumers on the PBS.

Stakeholder feedback has been integral to the development of the program, and the Department of Health and Aged Care will continue to seek feedback during 2023 to support program rollout.

[22] Budget 2023-24 – budget.gov.au/content/bp2/download/bp2_2023-24.pdf

Recommendation 12

The Committee recommends that the Therapeutic Goods Administration make the following changes to its Orphan Drugs Program:

- **Provide automatic access to the Priority Review Pathway for all medicines granted an orphan drug designation**
- **Treat paediatric patient populations as separate to adult patient populations for the purposes of the eligibility criteria**
- **Better account for the extra costs incurred by a sponsor in expanding its medicine to paediatric indications, for the purposes of assessing commercial viability as part of the eligibility criteria**
- **Where the prevalence of a disease is unknown in Australia, accept evidence of prevalence in other comparable countries or, in diseases of extremely low prevalence, worldwide for the purposes of the eligibility criteria.**

The Australian Government accepts this recommendation in part.

Automatic Priority review of orphan drugs

The Australian Government does not accept this part of the recommendation.

Current arrangements allow assessment of medicines to be undertaken as a priority where:

- the medicine is a new prescription medicine OR a new indications medicine; AND
- an indication of the medicine (the priority indication) is the treatment, prevention or diagnosis of a life-threatening or seriously debilitating condition; AND
- either:
- no therapeutic goods that are intended to treat, prevent or diagnose the condition are included in the ARTG (except in the part of the ARTG for goods known as provisionally registered goods); OR
- if one or more therapeutic goods that are intended to treat, prevent or diagnose the condition are included in the ARTG (except in the part of the ARTG for goods known as provisionally registered goods) – there is substantial evidence demonstrating that the medicine provides a significant improvement in the efficacy or safety of the treatment, prevention or diagnosis of the condition compared to those goods; AND
- there is substantial evidence demonstrating that the medicine provides a major therapeutic advance.

There is a significant equity risk in expediting medicines for rare conditions but not those that treat larger populations, especially in cases where the orphan drug may not represent a significant therapeutic advance. As well as additional and unfunded impact on the TGA's resources, priority evaluations can place a significant burden on sponsors, not all of whom are able to provide the rapid responses necessary to meet the required timeframes.

Automatically assigning priority status to all orphan medicines will substantially increase the number of priority medicines and cause additional unfunded costs to the TGA, and further Australian Government consideration of funding options would be required.

Treat paediatric populations separately to adult populations

The Australian Government does not accept this part of the recommendation.

Paediatric patient populations are already considered separately to adult patient populations when the disease process is considered to be different in the paediatric population or is specific to the paediatric population.

If paediatric populations were considered to be a separate population in all cases for the purposes of orphan medicine designation, Australia's approach would no longer align with international approaches such as those used by the European Medicines Agency and would hinder international collaboration. In addition, the basis of the orphan designation is to provide an incentive for regulatory submissions if the entire treatment population is small; separating out adult and paediatric populations (which when considered together may form a substantial population) does not align with that logic.

Changing the criteria would result in an increase in applications. As orphan drug reviews are not cost recovered, further consideration of funding options would also be required.

Expanding medicines to paediatric indications

The Australian Government accepts this part of the recommendation in principle.

To support availability of paediatric specific formulations (e.g. syrup), the orphan drugs program already has a separate pathway for new dosage form medicines. This pathway is intended to be an incentive to sponsors to register new dosage form medicines that would not be financially viable in the absence of a TGA fee waiver. In this pathway, the medical plausibility and prevalence criteria do not apply for orphan designation. However, the existence of this pathway may need to be publicised more to sponsors.

Financial viability, along with other criteria, can also be considered within the general orphan designation criteria for a medicine that is not a new dosage form.

Evidence of prevalence

The Australian Government accepts this part of the recommendation.

The TGA already accepts evidence to support prevalence based on epidemiological data or databases available overseas supplemented with an explanation of the extrapolation to the Australian population. This is currently included in the orphan drugs program guidance.[23]

[23] www.tga.gov.au/publication/orphan-drug-designation-eligibility-criteria

Recommendation 13

The Committee recommends that the Department of Health reform its regulatory and reimbursement processes to enable therapeutic goods to be registered and reimbursed by molecular indication in addition to by disease indication. This should include legislative change if necessary.

The Australian Government accepts this recommendation.

The TGA processes for prescription medicine approval already allow for registration of a medicine based on a molecular indication, i.e. they are 'tissue agnostic'. Molecularly defined indications for use of medicines are used to either further characterise a histology-based condition, or where there is a molecularly-defined abnormality occurring in more than one organ, permitting a histology-agnostic indication. For example, larotrectinib (Vitrakvi®), which is mentioned in the Inquiry Report, is currently provisionally registered in adults and paediatric patients with locally advanced or metastatic solid tumours based on a molecular indication, i.e. neurotrophic tyrosine receptor kinase gene fusion. Pembrolizumab (Keytruda®) also has two provisionally registered indications that are tissue agnostic (unresectable or metastatic solid tumours that are microsatellite instability-high or mismatch-repair deficient or mutational burden-high solid tumours).

However, some medicines which may qualify for orphan designation based on a specific disease may be less likely to meet orphan requirements with a molecular indication.

The process for subsidising medicines through the PBS allows for medicines to be subsidised by molecular indication where this is supported by the evidence presented in the submission. Larotrectinib, for example, is subsidised for children for the treatment of solid tumours of any type who have the neutrophic tropomyosin receptor kinase gene fusion.

The decision to recommend reimbursement of a medicine on the PBS for a histology-specific or molecularly-based indication will depend on the codependency claim for the molecular test and therapy, and also the clinical safety and effectiveness data presented for assessment. When a predictive molecular test that defines the codependency becomes universally utilised in patients with histological diagnoses, the assessment of codependency for therapy may no longer be required, based on evidence presented to support a reimbursement application.

Recommendation 14

The Committee recommends that the Australian Government reconsider the current cost recovery funding model for the Therapeutic Goods Administration, paying attention to future staffing and IT infrastructure needs in an environment where demand on its services and systems are expected to increase in future years. The Committee recommends funding specifically for:

- **IT systems upgrades, to modernise and match the IT capability of other overseas Tier 1 regulators.**
- **An expansion of its staffing capacity in areas of new medical and technological advances including for horizon scanning.**
- **The release of TGA Australian Public Assessment Reports at the same time as a prescription medicine is listed.**
- **The implementation of the HECS-style fee waivers outlined in Recommendation 8.**

The Australian Government accepts this recommendation.

In recent months, the Department of Health and Aged Care has undertaken work on the TGA funding model, supported by external consultants. This includes an assessment of the appropriate levels of cost recovery for services to industry as well as an identification of public health/public good and other activities that may not be appropriate to cost recover from industry.

The TGA's public health/public good activities, which include both the specific elements identified in the recommendation as well several other areas, currently cost almost $30 million per year. These activities include compliance, enforcement and litigation costs (of which 90 percent of the effort is for products not approved by the TGA), consumer and health professional education and communication activities, advisory support for researchers and small businesses, as well as the list of activities proposed by the Committee. The TGA currently receives a limited appropriation to support certain activities such as the orphan drug program, the COVID-19 response and the Special Access Scheme.

As part of the Budget 2023-24, TGA will receive funding for four years for activities aimed at protecting public health ($61 million over four years) which includes, but is not limited to, compliance and enforcement for products and companies outside the regulatory system, increased engagement and communication with consumers and healthcare professionals and continued assistance to small and medium enterprises particularly those developing emerging technologies.

The TGA is currently implementing a consolidated workflow management system, which includes time tracking, to clearly determine the costs of chargeable and non-chargeable

activities in accordance with the Australian Government Charging Framework.[24] Following completion of a multi-month time tracking exercise, the Australian Government will consider any changes to current funding arrangements.

[24] www.finance.gov.au/government/managing-commonwealth-resources/implementing-charging-framework-rmg-302/australian-government-charging-policy#australian-government-charging-framework

Recommendation 15

The Committee recommends that the Australian Government ensure the membership of the Pharmaceutical Benefits Advisory Committee and Medical Services Advisory Committee provides the appropriate expertise for all applications. This should include the possibilities of enhanced cross-membership between the two committees and the appointment of temporary members to consider individual applications.

- **Recognising the nature of health challenges in Indigenous communities, membership should include representation from Aboriginal and Torres Strait Islander Peoples.**

The Australian Government accepts this recommendation in principle.

The Department of Health and Aged Care and the HTA Committee chairs remain committed to accessing people with the skills to provide the best advice and facilitate cross-member interaction and information sharing. It is not feasible for a standing committee to hold expertise for every specialised application it receives, and the committees are able to source external expertise to supplement the expertise of their standing membership, including when dealing with very rare diseases or specialised therapies.

Cross-membership of HTA Committees continues to be a feature of the existing committee structure. There are also several committee members who are appointed to more than one HTA Committee or sub-committee. Further, codependent applications are considered jointly by members from the MSAC Evaluation Sub-committee (MSAC ESC) and the PBAC Evaluation Sub-committee (PBAC ESC).

The Australian Government recognises the nature of health challenges in First Nations communities and agrees with the objective that membership of MSAC and PBAC should include representation from First Nations peoples. The MSAC includes a First Nations member.

As part of this objective, the Department of Health and Aged Care will continue to consult with First Nations organisations when seeking nominations for MSAC, PBAC and their sub-committees.

Current committee members have extensive experience in work relevant to First Nations communities, including experience with:

- the Council of the National Health and Medical Research Council (NHMRC)
- the National Aboriginal Community Controlled Health Organisation
- the Poche Centre for Indigenous Health
- the Advisory Group of the University of New South Wales Research Centre for Primary Health Care and Equity (for which Indigenous Health is one of the three research streams)
- the Townsville Aboriginal and Islander Health Service

- the Australian Institute of Aboriginal and Torres Strait Islander Studies research ethics committee.

In 2020, all Australian governments signed the National Agreement on Closing the Gap, negotiated in genuine partnership with First Nations representatives. The National Agreement on Closing the Gap includes four Priority Reforms that commit all Governments to changing the way they work with First Nations people.

The Department of Health and Aged Care has established a Closing the Gap Steering Committee to drive its implementation of the Priority Reforms. The Closing the Gap Steering Committee will undertake a review of the Terms of Reference of all departmental committees to ensure they align with the Priority Reforms, which includes a measure of the number of First Nations representatives on boards and committees.

Recommendation 16

The Committee recommends that the Department of Health investigate further opportunities for the formation of an international Health Technology Assessment consortium similar to the Access Consortium to streamline the regulatory process for certain medicines and medical technologies. This investigation should include discussions with representatives of the Health Technology Assessment bodies of the United Kingdom, Canada and other countries with systems similar to Australia's.

- **The Committee recommends that the Therapeutic Goods Administration work with the United States Food and Drug Administration and other overseas regulators to establish an equivalent of Project Orbis for non-cancer rare diseases, or to expand Project Orbis to include such diseases.**

The Australian Government accepts this recommendation.

Increased international collaboration has the potential to benefit Australians through the ability to support reduced market authorisation timeframes, support the TGA to make informed and internationally consistent decisions about therapeutic products, and reduce regulatory duplication for industry. For regulatory review, there are major work sharing programs involving the ACCESS Consortium,[25] bilateral collaboration with the European Medicines Agency, and extensive acceptance of Comparable Overseas Regulator Reports.

The TGA approached the United States Food and Drug Administration (US FDA) in 2019 to discuss the potential for expanding Project Orbis into areas other than oncology, but at that time this was not supported by the US FDA. The TGA will continue to engage with international regulatory partners and industry to seek opportunities for further collaboration on shared evaluations, as there have been recent suggestions of interest in collaboration on cell and tissue therapies.

In May 2021, the Department of Health and Aged Care signed a Memorandum of Understanding with the Singapore Ministry of Health in the field of health cooperation, which will facilitate deeper exchanges of information, knowledge and expertise in health technology assessment.

Further, the Minister for Health and Aged Care has recently approved the Department of Health and Aged Care, together with the PBAC and MSAC, to enter into a collaboration arrangement with like-minded HTA agencies in the United Kingdom and Canada. Additional Canadian and New Zealand HTA agencies joined the collaboration in September 2023.[26] The collaboration arrangement will provide a framework for close and collaborative ways of working to support strategic objectives, which includes collaboration with these agencies to identify and progress opportunities to improve HTA and regulatory collaboration.

[25] www.tga.gov.au/australia-canada-singapore-switzerland-united-kingdom-access-consortium
[26] www.pbs.gov.au/info/news/2023/09/international-hta-collaboration-expands

The Australian Government acknowledges the potential for streamlined assessment processes for reimbursement decisions through work sharing with countries that have similar systems to Australia. The terms of reference for the HTA Review include in its scope consideration of the feasibility of international work sharing for evaluation of technologies in scope for the HTA Review.

Recommendation 17

The Committee recommends that the Australian Government establish a scheme that supports the domestic medical technology sector, similar to the Food and Drug Administration's Breakthrough Devices Program in the United States.

The Australian Government accepts this recommendation in principle, noting that further investigation is required (including broad stakeholder consultation and consideration of funding options).

Existing TGA pathways for priority applications for new and novel devices could be enhanced subject to availability of resources. These pathways currently focus on the late development stage of medical devices (including medical devices already approved in other regulatory jurisdictions), and offering shorter timeframes for regulatory evaluation of medical devices.

Should an early development stage program for medical devices be established, assessment by the TGA could focus on examining the 'breakthrough' aspect of the technology, requiring the likelihood of major clinical advantage (i.e. based on pre-clinical evidence or pilot stage single-arm clinical evidence).

A Review of the National Medicines Policy was undertaken during 2021 and 2022 in recognition of the substantial changes to the health landscape since the original policy was published in 2000.

Stakeholder feedback from the Review's consultation processes called for a continued focus on supporting thriving, dynamic medicines industry and research sectors with the capability, capacity and expertise to respond to current and future health needs.

An updated National Medicines Policy was published in December 2022.[27] Promoting responsive, innovative and sustainable medicines industry and research sectors is one of its central themes.

In addition, the Government's $15 billion National Reconstruction Fund (NRF) will facilitate increased flows of finance into priority areas of the Australian economy, through targeted investments to diversify and transform Australian industry, create secure, well-paying jobs, and boost sovereign capability.

The Government has earmarked $1.5 billion of NRF finance for the medical science priority area. This will build Australian medical manufacturing including in medical devices, medicines and vaccines. The NRF also includes $1 billion of finance earmarked to grow Australia's advanced manufacturing capability across the priority areas and encourage the commercialisation of home-grown innovation and technology.

[27] www.health.gov.au/resources/publications/national-medicines-policy

Recommendation 18

Recognising the vital role that vaccines play in addressing many diseases, including its importance in providing protection against Covid-19, the Committee recommends that the Department of Health conduct a review of the National Immunisation Program. This review should focus on reforming existing approaches used to value vaccines to ensure early and rapid deployment of vaccines in Australia.

The Australian Government notes this recommendation.

The NIP is a collaborative initiative involving all levels of government, healthcare providers, administrators and researchers. It provides free vaccines across 17 disease groups to eligible people including infants, children, young adults, vulnerable adults and older people who are at greater risk of serious harm from vaccine preventable diseases. In order to list a vaccine on the NIP, under legislation it must be approved by the TGA and considered and recommended by the PBAC.

As noted in response to Recommendation 3, the Strategic Agreement between the Commonwealth and Medicines Australia includes a commitment to an HTA Review. Australia's health technologies policy includes the range of processes and mechanisms that use scientific evidence to assess the quality, safety, efficacy, effectiveness and cost effectiveness of health services in Australia, including vaccines. As part of the review process, the Reference Committee for the HTA Review will consider a number of matters, including the methods for evaluating vaccines for listing on the NIP and the suitability of existing funding pathways as required.

The Australian Government agrees that vaccine assessment processes, including the purchase of vaccines through NIP, should be fit for purpose for Australian health needs. This includes accounting for the innovation involved in producing vaccines for specific or novel strains of diseases where existing treatment options are limited, the level of efficacy of vaccines in comparison with existing treatments, and reducing the time taken for assessment of new vaccines.

The NIP is not intended to procure or deploy vaccines in a national health emergency situation. During the COVID-19 pandemic, vaccine procurement was managed under item 182 of Schedule 1AB of the Financial Framework (Supplementary Powers) Regulations 1997.[28] This item allows financial support for activities that strengthen the nation's capacity and capability to detect, prepare for and respond to health emergencies and communicable diseases.

The Australian National Audit Office undertook a review to assess the effectiveness of the planning and implementation of the COVID-19 vaccine rollout, which was tabled in August 2022. The Department of Health and Aged Care welcomed the findings and

[28] www.legislation.gov.au/Latest/F2022C00438

recommendations in the report, and has already commenced steps to address the issues identified in this audit. [29]

[29] www.anao.gov.au/work/performance-audit/australia-covid-19-vaccine-rollout

Recommendation 19

The Committee recommends that the Australian Government continue to address the following matters in its reforms to the Prostheses List:

- **The lack of coverage for non-implantable devices under the current arrangements.**
- **Improving coordination between the Medical Services Advisory Committee and the Prostheses List Advisory Committee to provide faster access for patients.**

The Australian Government accepts this recommendation and will continue to consider these matters in its reforms to the Prescribed List of Medical Devices and Human Tissue Products (formerly the Prostheses List).[30]

[30] Further information on these reforms is at www.health.gov.au/health-topics/private-health-insurance/the-prostheses-list/prostheses-list-reforms-and-reviews

Recommendation 20

The Committee recommends that the Australian Government establish a last resort mechanism for directly securing ongoing supply of medicines that meet a high clinical need and lack suitable alternatives that are at risk of being delisted from the Pharmaceutical Benefits Scheme.

The Australian Government accepts this recommendation.

When a pharmaceutical company submits a request to remove a medicine from the PBS, advice is often sought from the PBAC.

Where the PBAC considers the delisting of a drug will result in unmet clinical need, the Australian Government works with the pharmaceutical company that supplies the medicine to consider options to retain it on the PBS. Pharmaceutical companies can also apply for price increases prior to submitting a delisting request, which are agreed to when necessary for continued supply of a clinically important medicine. If the listing cannot be maintained, the Australian Government implements management actions to minimise the impact of the delisting.

Where delistings result in medicine shortages and an overseas registered medicine has been approved for supply under section 19A of the Therapeutic Goods Act, the Australian Government reaches out to pharmaceutical companies and works with them to subsidise that brand through the PBS. In the event of a serious shortage, the TGA may also publish a Serious Scarcity Substitution Instrument (SSSI) which allows community pharmacists to substitute specific medicines without prior approval from the prescriber. The Australian Government has implemented changes that allow PBS subsidy in circumstances where the TGA has issued a SSSI.

Furthermore, the Australian Government is considering options to directly seek expressions of interest for medicines where the PBAC considers that the delisting of a drug, or a form of a drug, will result in unmet clinical need and no other brands remain listed on the PBS.

Medicine shortages and delistings occur for many reasons and unfortunately, an uninterrupted supply chain can never be guaranteed. The Australian Government administers several policies to ensure pharmaceutical companies supply PBS listed medicines where possible. Pharmaceutical companies are required to provide an assurance of supply for newly listed products, undertaking that sufficient stock of the product to meet demand will be available in time for the first PBS listing day. In addition, Guarantee of Supply requirements outlined in Division C, Part VII of the National Health Act apply to newly listed brands that are bioequivalent or biosimilar to an existing listed brand and where a pharmaceutical company offers a lower price. The Guarantee of Supply period ends after 24 months or when another brand assumes the obligation.

The Commonwealth has entered into Strategic Agreements with Medicines Australia and GBMA. These Agreements include a Medicines Supply Security Guarantee to bolster medicine supply to Australian patients. The Medicines Supply Security Guarantee is

designed to help protect Australian patients, pharmacists, and prescribers from the impact of the increasing number of global medicines shortages by implementing mandatory stock levels for certain critical high-volume medicines. Under amendments to the National Health Act that received Royal Assent on 16 December 2021, pharmaceutical companies are required to hold four to six months of stock for 'designated brands' in Australia. These amendments came into effect from 1 July 2023.[31]

[31] *National Health Amendment (Enhancing the Pharmaceutical Benefits Scheme) Act 2021*, www.legislation.gov.au/Latest/C2021A00139

Recommendation 21

The Committee recommends:

- **The federal, state and territory health authorities complete the standardisation of newborn screening across Australia.**
- **As part of that process, the Australian Government work with states and territories to expand the newborn screening program based on new understandings of genomic testing for conditions and international best practice.**
- **That the Australian Government in collaboration with states and territories, conduct reviews every two years to determine whether the screening program should be further expanded based on new Australian and international scientific and medical knowledge.**

While not in the terms of reference for this inquiry, the Committee recognises and supports the calls from rare disease patient groups for more funding for treatment pathways for actionable disorders across states and territories, where identified through newborn screening.

The Australian Government accepts this recommendation in principle.

The 2022-23 October Budget provided funding of $39.0m over four years to increase the number and consistency of conditions screened in Australia's newborn bloodspot screening (NBS) programs and develop a repeatable condition identification assessment process to assess new conditions.

The Australian Government has made significant progress towards delivering on this commitment.

On 29 March 2023, the Commonwealth Health Minister offered funding of $25.3 million to states and territories to support expansion, through a schedule to the Health Federation Funding Agreement. Through this partnership, the Commonwealth and states and territories will work collaboratively to screen babies consistently.

The *Newborn Bloodspot Screening National Policy Framework* (NBS Framework) describes the current national decision-making criteria to assess conditions for inclusion or exclusion in NBS programs.[32] A national genomics position statement has also been developed which provides guidance on the use of genomics in population screening.[33] The Australian Government, together with states and territories, will review the NBS Framework and position statement in light of its commitment to expand NBS programs, and will continue to monitor emerging evidence on genomics to inform further changes into the future.

The Australian Government also contributes to services within public hospitals, including NBS, through the NHRA. Under the NHRA, the Australian Government provides a

[32] www.health.gov.au/resources/publications/newborn-bloodspot-screening-national-policy-framework
[33] www.health.gov.au/resources/publications/genomic-tests-in-population-based-screening-programs-position-statement

significant funding contribution to assist states and territories with the costs of delivering public services through their health and hospital systems. The Australian Government's funding contribution for public hospital services to the states and territories under the NHRA was $29.6 billion in 2021-22, which includes the funding paid through the National Partnership on COVID-19 Response. This funding is estimated to grow to $32.6 billion in 2025-26.

The Australian Government notes the Committee's call for increased support for treatment pathways for babies diagnosed with a disorder through NBS. All population-based screening programs must have adequate facilities for follow-up assessment, diagnosis, management and treatment prior to being recommended for inclusion in a screening program. The Australian Government, in collaboration with states and territories, will consider these clinical management pathways in developing implementation arrangements.

Recommendation 22

The Committee recommends that all levels of government prioritise and implement with urgency the harmonisation of Human Research Ethics Committee (HREC) and Site-Specific Assessment submissions into one Australian online platform and enable parallel review by HRECs and Research Governance Offices.

- **The platform should be developed within the purview of the Australian Commission on Safety and Quality in Health Care.**
- **This work should be a continuation from the work prepared as part of the National Clinical Trials Governance Framework.**

The Australian Government accepts this recommendation in principle.

Work is underway to harmonise Human Research Ethics Committee (HREC) and Site-Specific Assessment submissions into one Australian online platform. Nation-wide consultations were undertaken on a cross-government 'National One Stop Shop' platform for Clinical Trials and Human Research Approvals, and the related National Clinical Trials Front Door. More than 5,000 individuals and groups informed this development process, including jurisdictional health departments, health service organisations, industry, universities, medical research institutes, government agencies, consumers, and other individuals. Stakeholder feedback demonstrated support for a single national platform that would replace existing systems and operate under the purview of the Department of Health and Aged Care.

The ACSQHC undertook consultations on the development of the proposed National One Stop Shop,[34] on behalf of the Department of Health and Aged Care and all jurisdictions. This builds on previous work on the National Clinical Trials Governance Framework.[35] The Consultation Report is available on the ACSQHC website[36].

The Australian Government announced on 31 October 2023 the appointment of Emeritus Professor Ian Chubb AC as Chair of the Inter-Governmental Policy Reform Group (IGPRG) for health and medical research. The IGPRG will provide policy and operational oversight of the National One Stop Shop for health and medical research.[37]

[34] www.safetyandquality.gov.au/our-work/health-and-human-research/national-one-stop-shop-national-platform-health-and-human-research
[35] www.safetyandquality.gov.au/standards/clinical-trials
[36] www.safetyandquality.gov.au/publications-and-resources/resource-library/consultation-report-national-one-stop-shop
[37] www.health.gov.au/ministers/the-hon-mark-butler-mp/media/eminent-australian-to-lead-one-stop-shop-clinical-trials-reform-group

Recommendation 23

The Committee recommends that all levels of government jointly provide funding for the development of a national clinical trial register. It should include:

- **Development of a sophisticated digital platform to collect and facilitate patient identification, patient recruitment, patient retention and completion rates for clinical trials.**
- **Linked data from existing national registers and consideration should be given to whether the register is best operated by a government agency or an existing Non-Government Organisation, or an academic body with appropriate experience.**

The Australian Government notes this recommendation.

As outlined under Recommendation 22, the Department of Health and Aged Care and the ACSQHC are currently exploring options for a national clinical trial register embedded into the proposed National One Stop Shop for Clinical Trials and Human Research Approvals.

There is support, including by all jurisdictions, to adopt an ambitious approach to development of the single Australian national approval platform (One Stop Shop). This is viewed as an opportunity for genuine transformative change that would address longstanding challenges to significantly improve Australia's current global positioning. It is expected to cover the whole life cycle, from point of first contact with a site, through pre- and post-approval processes, to post-trial monitoring.

There is sector support for a national health and medical research registry to be embedded into the national approvals platform and information workflow, to ensure the quality and currency of information. It will also be important that the One Stop Shop be designed to enable a range of other initiatives across the sector, where appropriate.

A range of views and innovative options have also been forthcoming in relation to design of the proposed National Clinical Trials Front Door. A key proposal explored as part of this process is the potential for a national recruitment portal – that is, the development of a sophisticated digital platform to facilitate patient identification and recruitment.

Recommendation 24

The Committee recommends the Australian Government develop policies that encourage modernising digital technologies and practices to position Australia as the premier destination for international clinical trials. This would include developing national standards for the use of e-consent, e-signature, and electronic medical records to enable remote monitoring and participation in clinical trials across Australia.

- **National standards should include standardising clinical costs and fees that are competitive with international fees.**

The Australian Government accepts part of this recommendation in principle and accepts its overall intent.

Digital technologies and practices

The Australian Government accepts this part of the recommendation in principle.

The Department of Health and Aged Care has already progressed a number of key projects and is continuing to develop further policies and a world leading cross-government platform, the National One Stop Shop for Clinical Trials and Human Research Approvals, to position Australia as a global leading destination for medical research including clinical trials.

The ACSQHC has developed the National Clinical Trials Governance Framework on behalf of the Department of Health and Aged Care and all jurisdictions.[38] The National Clinical Trials Governance Framework requires health service organisations to adhere to national policies and procedures and to use national systems (where they exist) to monitor and report on their clinical trial operations. This includes adherence to the National Standard Operating Procedures for Clinical Trials, including teletrials, which provide guidance on the use of e-consent, e-signatures and electronic medical records.

From 2023, health service organisations will be assessed against specific actions in the National Safety and Quality Health Service Standards, as provided in the National Clinical Trials Governance Framework, and the accreditation of health service organisations for the provision of clinical trial services will be incorporated into the Australian Health Service Safety and Quality Accreditation Scheme.

The National Standard Operating Procedures for Clinical Trials, including teletrials, and the National Principles for Teletrials in Australia both form part of a Teletrials Compendium (the Compendium),[39] developed in collaboration with jurisdictions and key government agencies to support a consistent national approach to implementation of teletrials in Australia. This will particularly support and enable clinical trials in rural, regional and remote areas.

[38] www.safetyandquality.gov.au/standards/national-clinical-trials-governance-framework
[39] www.health.gov.au/resources/collections/the-national-teletrials-compendium

The Compendium has been developed to assist organisations engaged in conducting clinical trials in Australia to, wherever possible, standardise their procedures for key operations related to clinical trials and specifically teletrials. They have been developed for the National Mutual Acceptance Scheme in Australia and also to support a consistent approach to national implementation more broadly.

Compliance with the Compendium provides public assurance that the rights, safety and wellbeing of trial participants are protected, consistent with the principles that have their origin in the Declaration of Helsinki,[40] and that the clinical trial data generated from the clinical trials are credible. The Compendium is also intended to conform with the Universal Declaration on Bioethics and Human Rights,[41] which seeks to address ethical issues related to medicine, life sciences and associated technologies as applied to human beings, taking into account their social, legal and environmental dimensions, and to provide guidance to decisions or practices of individuals, groups, communities, Institutions and corporations, public and private.

Further, the Compendium is consistent with the National Statement on Ethical Conduct in Human Research (the National Statement),[42] and aligns with the National Clinical Trials Governance Framework which has been designed to support the delivery and integration of high-quality clinical trials service provision into routine hospital care for improved patient outcomes. The National Principles for Teletrials are also consistent with recommendations from the Clinical Oncology Society of Australia's *Australasian Teletrial Model – A National Guide to Implementation* (September 2016).[43]

The Compendium has been endorsed by all Australian states and territories, together with the TGA and the NHMRC, through the Clinical Trials Project Reference Group. It applies to all health service employees including, but not limited to, visiting health professionals, contractors, consultants and volunteers who propose to undertake, administrate, review and/or govern human research involving patients/participants, facilities and or staff. It is understood that all study personnel involved in the clinical study must operate within their scope of practice.

The standard operating procedures include information about the use of e-consent, e-signatures and electronic medical records. E-consent should be considered as an option for teletrials as consent signatures can be obtained contemporaneously at both Primary and Satellite Sites. This will help to ensure consistency of practice with the essential elements of consent outlined in the National Statement, Chapter 2.2 and the

[40] World Medical Association Declaration of Helsinki – Ethical Principles for Medical Research Involving Human Subjects www.wma.net/policies-post/wma-declaration-of-helsinki-ethical-principles-for-medical-research-involving-human-subjects/

[41] en.unesco.org/about-us/legal-affairs/universal-declaration-bioethics-and-human-rights

[42] www.nhmrc.gov.au/about-us/publications/national-statement-ethical-conduct-human-research-2007-updated-2018

[43] www.cosa.org.au/media/332325/cosa-teletrial-model-final-19sep16.pdf

International Council for Harmonisation (ICH) Guideline for Good Clinical Practice (GCP) E6 (R2) Section 4.8.10.[44]

More broadly, the Australian Government is working to support information sharing across the health system, both for near real time clinical information and data sets to inform population health measures. The Australian Digital Health Agency is responsible for delivery of My Health Record and the National Clinical Terminology Service, as well as developing the National Healthcare Interoperability Plan, to further support safe and secure information sharing between healthcare providers.

The Australian Digital Health Agency, the Department of Health and Aged Care and the Australian Institute of Health and Welfare are working in partnership to implement the framework for research and public health use of My Health Record data. Work to implement the framework is currently underway and includes a refresh of the governance arrangements and establishing the end to end systems, processes, big health data analytics and sharing capabilities, de-identification as well as individuals' consent model.

The program envisages a dynamic and enduring consent model and will inform the use and sharing of health information for research and public health purposes, which includes participation in clinical trials nationally in Australia. This work could also inform the development of future data registries such as a high-risk implantable device registry that captures the GS1 barcodes of implanted devices that appears in a patient's My Health Record and could be monitored with the appropriate permissions.

Clinical trial costs

The Australian Government accepts the intent of this part of the recommendation, noting the limitations outlined below.

In 2015 the Independent Hospital Pricing Authority developed a robust set of standard costs for items associated with conducting clinical trials in Australia, including standard costs for drug manufacturing, preparation and dispensing.[45]

The determination of standard costs associated with clinical trials in Australia is seen as a starting point to negotiate price. Ultimately, sponsors, trial sites and jurisdictions determine its appropriate application.

[44] ichgcp.net

[45] www.ihacpa.gov.au/resources/development-table-standard-costs-conducting-clinical-trials-australia

Recommendation 25

The Committee recommends the Australian Government should develop a national standard approach, including nationally agreed systems and standard operating procedures to support and strengthen the capacity to conduct clinical tele-trials in rural, regional and remote areas.

- **This approach should be developed in consultation with industry and allied health workers.**
- **This would include the need for education and training opportunities for General Practitioners and all allied health workers engaging in clinical trials using tele-trials and multi-centre trials.**

The Australian Government accepts this recommendation and agrees that a national standard approach, including nationally agreed systems and standard operating procedures, will support and strengthen the capacity to conduct clinical teletrials in rural, regional, and remote areas.

As outlined in response to Recommendation 24, significant work has already been undertaken in this regard, including the development and implementation of the National Clinical Trials Governance Framework and development of the Compendium which covers principles and standard operating procedures for clinical trials and teletrials.

The National Clinical Trials Governance Framework was endorsed for implementation by all jurisdictions in February 2022 and is recognised as a significant reform for the clinical trials sector. It embeds clinical trials into routine health service provision and strengthens the clinical and corporate governance arrangements for governments, hospital administrators, health services, private companies, trial sponsors and trial investigators in metropolitan, rural and remote locations that deliver clinical trials.

The Compendium has been agreed by all Australian states and territories, the TGA and the NHMRC through the Clinical Trials Project Reference Group and is available on the Department of Health and Aged Care website. The Compendium is consistent with minimum standards imposed by the ICH Guideline for GCP E6 (R2), an international ethical and scientific quality standard for the design, conduct, performance, monitoring, auditing, recording, analysis and reporting of clinical trials that involve participation of humans.

The need for education and training opportunities for general practitioners, nurses and allied health workers engaging in clinical trials using teletrials and multi-centre trials is well documented and well understood. This work is being promoted through a number of initiatives, including the National Clinical Trials Governance Framework and the National One Stop Shop for Clinical Trials and Human Research Approvals.

As outlined in response to earlier recommendations, the ACSQHC has conducted national consultations on the National One Stop Shop for Clinical Trials and Human Research Approvals. These consultations are on behalf of all jurisdictions to scope the requirements for a cross-government national health and human research approvals

platform – a National One Stop Shop. The aim of the National One Stop Shop is to make it easier for patients, researchers, industry representatives and sponsors to find, conduct, participate and invest in high quality and ethical research in Australia.

The MRFF's National Critical Research Infrastructure (NCRI) initiative is funding research to establish and extend infrastructure of critical importance, including facilities, research equipment, systems and services that will be utilised to conduct world-class health and medical research. The 2019 Rural, Regional and Remote Clinical Trials Enabling Infrastructure Grant Opportunity was launched under the NCRI Initiative and provided $125 million over five years from 2020-21 to three projects to improve access to clinical trials for Australians living in rural, regional and remote areas.

Recommendation 26

The Committee recommends the Australian Government should continue to fund Clinical Trial Networks with a particular focus on developing seed funding for Indigenous Health Clinical Trial Networks.

The Australian Government accepts this recommendation in principle and acknowledges the important work of the Clinical Trials Networks (CTNs).

The Australian Government will explore the most effective ways to support CTNs to strengthen sector capability and collaboration with the aim of embedding evidence-based care in the health system. This includes meeting the needs of, and achieving outcomes for, First Nations people and communities as a critical aspect of Closing the Gap, and to progress the Australian Government agenda of increased high quality clinical trial activity that is conducted ethically and for the benefit of the Australian healthcare system and the users of that system.

Recommendation 27

The Committee recommends the Australian Government reform data exclusivity provisions in Australia with a view to extending data exclusivity for orphan drugs and vaccines to a period of up to 10 years. The Australian Government should:

- **Develop additional reforms to data exclusivity timeframes to support research and development into new drugs and novel medical technologies in areas of unmet need.**
- **Consider future funding initiatives for novel drug discovery and support research and development partnerships in Australia. This would assist new drugs and novel medical technologies in early stage and pre-commercial development.**
- **In partnership with the states and territories, develop and implement a pilot scheme for value-based payments for new antimicrobial drugs. This pilot should apply the lessons learned from the Australian Government's pilot scheme for payment for Hepatitis C drugs, as well as from overseas antimicrobial drug schemes.**
- **Promote the recent research and development tax initiatives internationally as a way of encouraging industry to look to Australia for future investments in the healthcare sector.**
- **Conduct a full review of the patent box scheme every two years after implementation to ensure it is operating effectively and driving increased expenditure and innovation within Australia.**
- **Collaborate with the states and territories to review the funding of the research and development sector in health care to distribute funding in a methodical way that provides sufficient support throughout the research funding 'pipelines'.**
    - **Noting the work underway through the Modern Manufacturing Program, the Committee supports the development of an updated roadmap to facilitate the manufacturing and commercialisation of novel drugs and technologies in Australia.**

The Australian Government notes this recommendation.

Australia's pharmaceutical intellectual property (IP) settings, including its data exclusivity regime, have been extensively reviewed over the previous decade, including most recently by the Productivity Commission (PC) in 2016. The PC found no evidence of systemic problems arising from Australia's current data exclusivity period and concluded that there are no grounds to extend the period of data protection for any pharmaceutical products beyond the current period of five years.[46] This accords with the earlier findings of the Pharmaceutical Patents Review (Patents Review) in 2013.[47]

[46] www.pc.gov.au/inquiries/completed/intellectual-property#report
[47] consultation.ipaustralia.gov.au/policy/pharma-patents-2013/

The Australian Government acknowledges the challenges associated with the development of orphan drugs and notes that individual countries have adopted a range of measures to encourage their development, including extending the time during which the sponsor enjoys market exclusivity. The Patents Review expressed doubts about whether this is an effective mechanism for addressing these challenges, given the relatively small size of the Australian pharmaceutical market. It considered that greater benefit may be derived from alternative measures, such as waiving or reducing TGA application fees, or subsidising or assisting research and development during the early and clinical trial stages. Currently, the TGA waives submission and evaluation fees for applications to register prescription medicines that have been designated by the TGA as orphan drugs, for as long as that designation remains in force. The Australian Government likewise waives PBAC submission fees for designated orphan drugs.

The Australian Government notes that Australia's data exclusivity regime fully complies with our international obligations, and that Australia's IP system ranks highly in international comparisons. The Australian Government is committed to ensuring that its pharmaceutical IP settings strike an appropriate balance between encouraging investment in the development of new, safe and effective medicines, and ensuring timely, affordable and sustainable access to such medicines for all Australians.

The Australian Government considers that additional periods of data exclusivity should be made available for orphan drugs and vaccines, but only where thorough analysis of the issue and evidence demonstrate this is necessary to encourage investment in these important areas. Such analysis would need to consider the potential impacts on access to medicines and the patent system, including the flexibilities available in that system, and the current and ongoing discussions on IP and vaccine accessibility occurring in international fora.

Australia's National Antimicrobial Resistance Strategy – 2020 and Beyond recognises that research into the development of new antimicrobial agents is a vital aspect in combatting antimicrobial resistance. Reflecting this, the Department of Health and Aged Care has commenced work towards identifying and scoping potential funding mechanisms and economic models that could be considered in the Australian context to incentivise the discovery of novel antibiotics and bring them to market in Australia.

The former Government announced that it would introduce a patent box for eligible corporate income associated with new patents in the medical and biotechnology sectors. The legislation for the medical and biotechnology patent box, the Treasury Laws Amendment (Tax Concession for Australian Medical Innovations) Bill 2022*,* was introduced into Parliament on 10 February 2022, and lapsed as a result of the 2022 Federal Election.

In the 2023-24 Budget, the Government announced it would not proceed with the patent box tax regime measures announced by the former Government. Targeted support for business investment will be provided through an enhanced Instant Asset Write off and the Small Business Energy Incentive.

From 2021 the Research and Development (R&D) Tax Incentive was enhanced to provide the Board of Industry Innovation and Science Australia with the power to make binding determinations about how it would exercise its powers and perform its duties.

Determinations provide companies with clarity and certainty and streamline access to the program. The first determination came into effect on 1 April 2022 and covers clinical trials.[48] It provides companies undertaking Phase 0, I, II and III, pre-market pilot stage and pre-market pivotal stage clinical trials for an unapproved therapeutic good with certainty that those activities are 'core R&D activities' under the program.

Austrade, in consultation with the Department of Industry, Science and Resources, continues to promote Australia as an attractive investment destination for companies developing new drugs and novel medical technologies. The Department of Industry, Science and Resources together with the Australian Taxation Office, which co-administer the R&D Tax Incentive, jointly brief Austrade on the R&D Tax Incentive requirements relevant to foreign entities, such as overseas activities and the clinical trials determination.

Further, as noted in response to Recommendation 1, the MRFF RCRDUN grant opportunities support clinical trials research that investigates new drugs, devices or treatments for rare cancers/diseases or for areas of unmet medical need.

While the Australian Government recognises the complexities and long-term challenges of investing in medical innovation, there are strong grounds for optimism. MTPConnect, the medical technology Industry Group Centre, has reported the continued growth of the sector across the key economic indicators, for example, in the five years to 2021:

- Gross value added per annum has risen an average of 2.3 percent per annum
- Jobs supported in industry and research have grown an average of four percent per annum
- Market capitalisation has grown an average of 21.9 percent per annum
- Capital raised has increased an average of 27.3 percent per annum
- R&D investment has increased an average of 13.9 percent per annum.[49]

[48] www.legislation.gov.au/Latest/F2022C00752
[49] www.mtpconnect.org.au/reports/SCP

Recommendation 28

The Committee recommends that:

- **The Department of Health integrate the patient voice upfront into the Health Technology Assessment system. Earlier patient engagement with the Health Technology Assessment system would include:**
    - **Representation from peak patient bodies that is refreshed every three – five years**
    - **Representation of Aboriginal and Torres Strait Islander Peoples.**
- **The Department of Health implement a notification system for all HTA bodies and the TGA to advise relevant patient groups of the receipt of an application.**
- **The Department of Health provide patients and stakeholders with a concise sponsor's submission summary to help facilitate their own involvement in the Health Technology Assessment process.**
- **The Department of Health should consider making patient evidence compulsory for certain applications, and should consider the role of patient evidence in the decisions of the Therapeutic Goods Administration.**
- **The Department of Health should notify relevant patient groups of the outcome of the assessment process by all HTA bodies.**
- **The Department of Health be funded to implement these recommendations.**
- **The Australian Government provide funding for organisations to support participation in the HTA process, including for very rare disease patient groups that have limited capacity for fundraising or access to alternative funding.**

The Australian Government accepts parts of this recommendation in full and other parts in principle.

Patient engagement in HTA

The Australian Government accepts this part of the recommendation. The Department of Health and Aged Care is committed to continuing engagement with consumers and integrating the patient voice into the HTA system.

Each Department of Health and Aged Care HTA Committee has at least one consumer committee member. These members do not represent peak bodies, though they may have been nominated by a particular organisation. Since 2021, four new consumer committee members have been appointed to HTA Committees for periods of between two and four years. Two of these four members have formal links with peak patient bodies although they do not represent these interests at the committee level. Mentoring programs have been trialled for new consumer committee members, with current consumer committee members providing input as to how best to attract and support consumers for future committee positions.

Consumer networks and patient organisations are encouraged to participate in workshops and forums for HTA capacity building, to gain experience with current HTA processes. Diverse representation is encouraged.

As noted in the response to Recommendation 15, the Department of Health and Aged Care continues to consult with First Nations organisations when seeking nominations for MSAC, PBAC and their sub-committees.

The Strategic Agreement between the Commonwealth and Medicines Australia includes a commitment that the Commonwealth and Medicines Australia will work to co-design and implement an Enhanced Consumer Engagement Process to capture consumer voices in respect of applications to the PBAC. It is intended that the process will capture the consumer and patient voice at an earlier stage of consideration of applications to list new medicines, or to expand indications for existing medicines. It is also intended that this process, once implemented, will be capable of informing reimbursement submissions for particular medicines. This work will complement continuing process improvements for when and how consumers are engaged and involved in HTA processes.

As part of *An Action Plan for Medical Devices*,[50] in 2020 the TGA established a consumer/patient working group comprising 21 representative bodies who have guided enhancements to processes and information for patients on medical devices. In June 2022 a Women's Health Products Working Group was established, focusing on both medicines and medical devices. Recommendations from this Group will assist the TGA in sourcing appropriate data or feed into efforts in a range of regulatory considerations, as well as research and registries. This Group will be useful for horizon scanning and advice as novel healthcare products seek approval to market and identify cross-cutting issues for consideration and application consistently across the regulatory review process.

Australia's National Medicines Policy emphasises the fundamental role of the consumer in achieving the policy's aim and intended outcomes. The National Medicines Policy highlights the importance of a person-centred, partnership approach that focuses on and responds to the needs of Australia's diverse population.

Notification of new applications

The Australian Government accepts part of this recommendation.

The Department of Health and Aged Care already uses an electronic notification process in the form of e-bulletins for both the PBAC and MSAC process. These bulletins include applications to be considered by HTA Committees and are disseminated at least six weeks before each meeting. Patient groups are also a focus in MSAC communications and consultation by the CEEU once an application has been deemed suitable to be considered by the MSAC. In addition to these activities, in 2023 the CEEU

[50] www.tga.gov.au/medical-devices-reforms-action-plan-medical-devices

has also initiated a more targeted communication to consumer and clinical groups once a PBAC agenda has been published.

Since January 2021 the TGA has published information on its website about new prescription medicine chemical entity and extension of indication applications received and under evaluation. The TGA will develop mechanisms for informing relevant patient groups of this information. Currently receipt of applications for new medical devices and IVDs is not publicly disclosed, and a decision of Government would be required for this long-standing policy to be changed.

The redevelopment of the TGA website will offer patients more options to subscribe to topics of their interest. A new electronic direct marketing solution will be implemented to offer patients direct messaging (via subscription) for recalls and safety information. The redeveloped website will use human-centred design to ensure the patient voice is captured in all decisions relating to the TGA website.

Submission summaries

The Australian Government accepts this part of the recommendation in principle.

The CEEU has been coordinating a pilot on the usefulness of plain English summaries of PBAC sponsor submissions since late 2020. The pilot aims to determine how consumers use these summaries to participate in HTA processes, what resources are required by industry to prepare these summaries, and what differences, if any, flow from consumers having access to these documents during the public consultation period. As part of this pilot, the CEEU has undertaken a range of consultation activities and this work will inform activity connected to the Strategic Agreement around enhancing consumer engagement processes as well as contributing to other international work on providing plain English summaries to consumers as part of HTA decision-making.

Patient evidence in PBAC and MSAC decisions

Patient evidence can be submitted in relation to items being considered by the PBAC and MSAC. The revised MSAC Guidelines[51] specifically include information for applicants on presenting patient evidence and highlight the importance of including patient-relevant outcomes. This includes outcomes that are directly relevant to the patient as well as those that reflect improvements in quality or length of life, family and societal outcomes and outcomes that relate to health disparities, such as equity of access, and areas of unmet clinical need.

[51] www.msac.gov.au/internet/msac/publishing.nsf/Content/Documents-for-Applicants-and-Assessment-Groups

Patient evidence in TGA decisions

The Australian Government accepts this part of the recommendation.

The TGA is better defining and communicating its use of Patient Reported Outcomes (PROs) and Real-World Evidence (RWE) following an external stakeholder consultation in 2021. A work plan has been published.[52] In 2022-23 the TGA will:

- establish a central point for information about RWE and PROs on the TGA website
- request that applicants document how RWE and PROs have been included in the application, and communicate when these are used in making regulatory decisions
- consult on adoption of internationally-relevant guidance and learn from international research for the use of RWE and PROs as evidence for the regulation of medicines and medical devices, covering generation of data and utilisation in evaluating the application. This is a developing field, and the TGA anticipates its guidance will be an agile resource for the industry
- support the enhanced use of RWE and PROs, potentially by providing advice to potential applicants and designers of RWE and PROs programs, and use for particular medicine regulation pathways
- encourage the adoption of medical device unique device identifiers in the broader healthcare system, and specifically in medical device registries, to support a stronger foundation for the collection of RWE and PROs.

In relation to medicines scheduling, assessment of benefits and intended use is already a mandatory factor in decision-making for scheduling, so applicants are already compelled to provide patient evidence. Most scheduling proposals involve two opportunities for public consultation and patient groups are notified about these consultations and subsequent scheduling decisions.

Notification of outcomes

The Australian Government accepts this part of the recommendation.

The Department of Health and Aged Care launched the HTA Consultation Hub on 1 December 2021.[53] This portal provides additional opportunities to engage with consumers and patient organisations, particularly to communicate outcomes to those who provided consultation input for a particular PBAC meeting. Discussions are underway as to how best to provide this information to patient groups. It is intended that this portal will also be used to provide additional information about MSAC outcomes, noting that MSAC public summary documents currently also include a plain English consumer summary.

[52] www.tga.gov.au/review-real-world-evidence-and-patient-reported-outcomes
[53] ohta-consultations.health.gov.au

Implementation funding

The Australian Government accepts this aspect of the recommendation in principle.

The Department of Health and Aged Care acknowledges the substantial time and resources required of consumer organisations to participate in HTA processes. Many consumer organisations are established to provide information and support to members and do not have the capacity to provide the type of input requested or to generate consumer evidence for HTA processes. The Department of Health and Aged Care has provided travel support to representatives from consumer organisations to attend face to face meetings and/or workshops related to HTA matters. Pilot activities to directly support consumer organisations to develop their comments for HTA Committee items are also in progress. The findings from these pilot activities will be captured by the implementation of the Strategic Agreement.

As noted in Recommendation 6 above, the HTA Consumer Consultative Committee was established in 2017, and the CEEU was established in 2019 to support broader consumer participation strategies across HTA. The CEEU has hosted a range of workshops and meetings with consumer organisations aimed at better supporting consumer representatives to engage effectively with HTA processes, including Consumer Symposiums aimed at improving engagement and communication with patients and consumer groups on HTA-related matters. Similar activities are planned across 2023 and will include strategies looking at how best to build the confidence and capacity of consumers and consumer organisations to contribute effectively to HTA processes.

Recommendation 29

The Committee recommends that:

- **The Committee recommends that the Australian Government amend the *National Health Act 1953* (Cth) to formalise the role and powers of the Pharmaceutical Benefits Advisory Committee Executive. The scope of the Executive's role and powers should be determined by agreement between the Executive and the Department of Health.**
- **The Department of Health produce a pre-submission advice framework for submissions to the Therapeutic Goods Administration, Pharmaceutical Benefits Advisory Committee, Medical Services Advisory Committee and other Health Technology Assessment bodies, explaining the interaction between those bodies and their evidentiary and other requirements, to be provided to sponsors before they make their submissions.**
- **The independent Health Technology Assessment Review reassess relevant aspects of the Health Technology Assessment process to ensure there are future pathways for treatments and therapies that do not fit neatly into the current system such as rare cancers, antimicrobials, orphan drugs, and precision medicines.**
    - **It is imperative that appropriate clear pathways are considered for inclusion for paediatric medicines and technologies.**
    - **The Committee is of the clear view that precision medicine approval pathways will require a different application assessment than current approaches designed for treatments for common conditions, with large data sets and comparative evaluations.**
- **The Department of Health publish data on application processing times and positive recommendation rates for the Pharmaceutical Benefits Advisory Committee and other Health Technology Assessment bodies. In addition:**
    - **The Department of Health should publish Health Technology Assessment processing times annually, benchmarked against other nations with advanced HTA processes.**
- **The Australian Government, in collaboration with relevant stakeholders, develop a suite of clear and measurable benchmarks to track the Commonwealth's implementations of the recommendations made by the Committee and accepted by the Australian Government.**
    - **These agreed benchmarks along with measurable KPIs/metrics should be developed in such a way as to best facilitate the Department of Health, including its agencies and other relevant statutory bodies, in the tabling of an annual update to the Australian Parliament.**

The Australian Government accepts this recommendation in principle, noting that changes to the National Health Act can only be made by the Australian Parliament.

Amendments to National Health Act

The PBAC Executive and Department of Health and Aged Care will consider options for formalising the role and powers of the PBAC Executive and provide advice to the Australian Government.

Pre-submission advice framework

Existing published guidelines provide submission frameworks for the TGA, PBAC and MSAC. The Department of Health and Aged Care will consider whether the existing information could be adapted.

The TGA has made regulatory pre-submission meetings available to applicants for many years, and in recent times has provided pre-submission advice to sponsors through joint meetings with other relevant HTA bodies within the broader Department of Health and Aged Care. This program of joint meetings will be expanded, although the emphasis of regulatory and HTA evaluation (and thus the issues to be addressed in pre-submission meetings) is often different. In addition, many HTA applications to MSAC focus on broader services and technologies rather than on the evaluation of products from individual sponsors.

The TGA is currently reviewing its guidance material for sponsors, in particular for pre-submission advice, although the work was delayed through diversion of resources during the COVID-19 pandemic. Regulatory guidance will also be better communicated following improvements to the TGA website, and new guidance on the use of RWE will be published. Website guidance to sponsors will also be developed to better explain the interaction between TGA regulatory evaluation and HTA assessment processes and their evidentiary and other requirements.

HTA Policy and Methods Review

The terms of reference for the HTA Review were prepared by the Reference Committee after it sought input from individuals and organisations representing patients, industry, clinicians, advisory committees, and Commonwealth and state and territory governments. The terms of reference for the HTA Review were published on 22 March 2023.[54] The terms of reference state that the HTA Review will consider HTA policy and methods for all medicines and vaccines, highly specialised therapies, and other health technologies (for example a pathology test or an imaging technology) that improve health outcomes associated with the aforementioned technologies. The technologies identified in this recommendation and associated policy and methods and funding and approval pathways will be considered by the HTA Review. It will be open to the Reference Committee to consider options for reform in these areas.

[54] www.health.gov.au/resources/publications/health-technology-assessment-policy-and-methods-review-terms-of-reference

Publication of performance data

The Department of Health and Aged Care currently publishes PBS process improvement metrics on the PBS website annually.[55] The first metrics report for Stage 1 PBS process improvements implemented on 1 July 2019 was published in August 2020. This includes metrics on Pre-Submission meetings, the Intent to Apply step, Notice of Intent for Pricing process and Pricing Pathways. Following implementation of Stage 2 PBS process improvements on 1 January 2021, a metric report on Stage 2 is also available, which reports on metrics in relation to the introduction of initial submission categories and resubmission pathways.[56] Further, the Medicine Status Website[57] became available in July 2019 to enable consumers to search for and monitor the status of medicines as they progress through the PBS listing process.

The Australian Government has committed to working with Medicines Australia to determine and make publicly available a range of key performance indicators on the time it takes for new medicines to be listed on the PBS including measures in control of the medicines industry (such as the time taken to make applications for regulation and reimbursement in Australia and in overseas jurisdictions) and measures in control of the Commonwealth (such as the time taken to assess applications).

The Department of Health and Aged Care is continuing to streamline and digitise the processes to support the public funding of health technologies through progressive implementation of and improvements to the Health Products Portal. Alongside this work, the Department of Health and Aged Care is developing a metrics framework to measure and report on PBAC assessments.

The Department of Health and Aged Care has collaborated with the Centre for Innovation in Regulatory Science (CIRS) since 2014 in its international HTA agency benchmarking study to monitor regulatory and HTA performance. The CIRS is an independent, not-for-profit organisation, which aims to advance regulatory and HTA policies and processes in developing and facilitating access to pharmaceutical products. As part of its ongoing benchmarking study, the CIRS publishes reports on nine jurisdictions (Australia, Canada, England, France, Germany, Poland, the Netherlands, Scotland and Sweden). Its latest report, *R&D Briefing 89; Review of HTA outcomes and timelines in Australia, Canada, Europe and the UK 2018-2022*, was published in September 2023.[58]

[55] www.pbs.gov.au/info/general/pbs-process-improvements

[56] www.pbs.gov.au/info/general/pbs-process-improvements

[57] www.pbs.gov.au/medicinestatus/home.html

[58] cirsci.org/publications/cirs-rd-briefing-89-review-of-hta-outcomes-and-timelines-in-australia-canada-europe-and-the-uk-2018-2022/

Reporting implementation of report recommendations

The Australian Government will consider appropriate reporting for implementation of review recommendations, noting many of the recommendations relate to existing initiatives that already have reporting mechanisms in place.

Recommendation 30

The Committee recommends that the Australian Government's independent Health Technology Assessment Review (which is scheduled to commence in July 2022) consider and develop reforms in the following areas:

- **Reducing the frequency and need for applications to HTA bodies to be resubmitted.**
- **Streamlining the interaction between hospitals and the Health Technology Assessment system**
- **Streamlining the interaction of the Therapeutic Goods Administration, the Pharmaceutical Benefits Advisory Committee, the Medical Services Advisory Committee and other Health Technology Assessment bodies**
- **Cooperation and harmonisation between Australian Health Technology Assessment bodies and equivalent bodies overseas**
- **Improving the measurement of the performance of the Pharmaceutical Benefits Advisory Committee and the publication of data on that performance**
- **Improving the mechanisms for communication between sponsors and the Pharmaceutical Benefits Advisory Committee during the submission process**
- **Increasing the use of Managed Access Programs to facilitate earlier access to innovative medicines**
- **Increasing the use of Real World Evidence in Health Technology Assessment**
- **Improving flexibility when choosing a comparator in Health Technology Assessment**
- **Introducing a scoping process that includes patients and clinicians at an early stage to agree on the framework that the submission will be considered. This process could draw on the approach taken by the United Kingdom's National Institute for Health and Care Excellence**
- **Improving the independent review process for HTA decisions, including the potential for this to be made available to groups of patients and clinicians in addition to sponsors.**

The Australian Government accepts this recommendation. The Strategic Agreement with Medicines Australia contains several commitments that address the areas mentioned in this recommendation.

As outlined above, the terms of reference for the HTA Review were published on 22 March 2023. The terms of reference were prepared by the Reference Committee in consultation with individuals and organisations representing broad range of interests.

The terms of reference states that the HTA Review will examine HTA policy and methods to identify features that:

- are working effectively
- may act as current or future barriers to earliest possible access
- may act as current or future barriers to equitable access

- detract from person-centredness
- may be creating perverse incentives.

The terms of reference for the HTA review states that it will consider reforms that address identified challenges and present a comprehensive set of recommendations for reforms to Government that:

- are implementable and sustainable for both health funders (Commonwealth, state, and territory) and the health technology industry
- deliver Australians equitable, timely, safe and affordable access to a high-quality and reliable supply of medicines for all Australians
- adopt a person-centred approach in HTA
- deliver the outcomes sought by recommendations from the Inquiry that are agreed in principle in the Government Response
- further the objectives of the new National Medicines Policy
- ensure HTA policy and methods are well adapted to and capable of assessing new technologies that are emerging or are expected to emerge in the coming years
- do not compromise assessment of patient safety, effectiveness and cost, or advice to Government on subsidy of health technologies.

The terms of reference encompass the areas identified in this recommendation. It will be open to the Reference Committee for the HTA Review to consider options for reform in the areas identified in this recommendation.

There are also further commitments in the Strategic Agreement for reform in the areas listed in Recommendation 30 that will occur during the term of the Strategic Agreement, informed by the outcomes of the HTA Review. These include:

- A commitment that improvements to HTA processes to facilitate earlier patient access to medicines will continue during the term of the agreement from 1 July 2022 to 30 June 2027. This will cover areas mentioned in this recommendation in relation to measurement of the performance of the PBAC and reducing resubmissions. The commitment states that these improvements will be informed by outcomes of Stage 2 PBS Process Improvements that were implemented at the start of 2021 and key metrics which will be co-designed and jointly agreed by the Commonwealth and Medicines Australia.

- A commitment that the Commonwealth and Medicines Australia work to co-design and implement an Enhanced Consumer Engagement Process to capture consumer voices in respect of applications to the PBAC. It is intended that the process will capture the consumer and patient voice at an earlier stage of consideration of applications to list new medicines or to expand indications for existing medicines. It is also intended that this process, once implemented, will be capable of informing reimbursement submissions for particular medicines.

- A commitment that the Commonwealth and Medicines Australia work together on conditional funding arrangements that use the current Managed Access Program

arrangements and establish transparent and robust criteria for reviewing existing funding arrangements for medicines and for managing exit from subsidy. The criteria are intended to improve the operation of these arrangements and greater use of these will be a measure of the success of this work.

Separate to the commitments in the Strategic Agreement, work to address some of the matters raised in this recommendation is already underway.

For example, the TGA and the PBAC have been streamlining interactions since 2020, to reduce the duplication and time taken between regulatory and reimbursement processes. New initial submission categories and resubmission pathways were introduced as part of PBS process improvements in January 2021 with the objective to reduce the number of resubmissions to the PBAC.

Sponsors of medical devices also have an option to submit Prescribed List (formerly the Prostheses List) applications for their devices in parallel with the assessment of their applications by the TGA.

Two major IT and business process improvement projects currently underway (TGA Digital Transformation and the Health Products Portal Project) will significantly improve the interaction through a single submission portal for regulatory and HTA consideration. The National HTA Chairs Committee is also currently exploring the benefits and need for cross-fertilisation and sharing of expertise between the various HTA Committees.

Recommendation 31

The Committee recommends that:

- **The Department of Health should consider, in consultation with state and territory governments, industry, patients and clinicians, the introduction of fees for Medical Services Advisory Committee applications on a cost recovery basis, if this is necessary to increase the speed and effectiveness of assessments. If fees are introduced they should have similar features to those recommended by the Committee for Pharmaceutical Benefits Advisory Committee fees (including those arrangements outlined at Recommendation 8). The Medical Services Advisory Committee increase the involvement of clinicians in its assessments of technologies with which its members lack relevant expertise.**
- **The Department of Health introduce an equivalent to the Managed Access Programs for medical devices. The details of this scheme including eligibility criteria and duration should be formulated in consultation with patient groups, clinicians and industry.**
- **The Therapeutic Goods Administration introduce parallel processing of applications with the Medical Services Advisory Committee.**
- **The Medical Services Advisory Committee increase opportunities for sponsors of particularly complex applications to present to it at its meetings and expand the opportunities for pre-submission meetings.**
- **The Medical Services Advisory Committee consider developing international collaboration for complex assessment proposals.**
- **The Department of Health expand the independent Health Technology Assessment Review in July 2022 to include Medical Service Advisory Committee processes.**
- **The Medical Services Advisory Committee publish a full calendar timeline of meeting agenda and outcomes, including dates when minutes and Public Summary Documents will be made public.**
- **The Medical Services Advisory Committee publish additional guidance for sponsors of digital health technologies.**
- **The Department of Health establish a benchmarking system for MSAC assessments, including benchmarking against comparable overseas organisations.**

The Australian Government accepts some parts of this recommendation in full and some parts in principle.

MSAC cost recovery

The Australian Government accepts this part of the recommendation. The Department of Health and Aged Care undertook a consultation process in 2022 regarding a proposal to implement a cost recovered HTA pathway and subsequent fees for applications to the MSAC. This proposal introduces cost recovery arrangements for all MSAC applications

from commercial entities seeking reimbursement decisions. The proposal is in line with the Australian Government Charging Framework. As part of the proposed cost recovery approach, applicants will be provided with greater clarity, transparency and certainty of timeframes for MSAC processes, similar to PBAC processes. Public consultation on this proposal took place in January and February 2022.[59] This consultation sought feedback on the cost recovery proposal and the associated regulatory impacts. The consultation outcomes will be considered alongside other relevant recommendations from this report (such as Recommendations 7, 8 and 31) and advice provided for Government consideration. The introduction of a cost recovery framework for MSAC activities was agreed in the 2023-24 Budget context.

Increased clinician involvement in MSAC

The Australian Government accepts this part of the recommendation. The MSAC's size and composition is determined by the Minister for Health and Aged Care. The MSAC's composition is drawn from a wide range of experts constituted from time to time to address the likely type of applications for the MSAC's consideration.

MSAC and its sub-committees are comprised of clinicians with a wide range of expertise, including in the areas of oncology, genetics, cardiology, pathology and endocrinology. MSAC may also co-opt non-members with specific skills and knowledge in an expert capacity to its sub-committees or working groups.

There are existing opportunities in the MSAC process that can be optimised to increase the involvement of clinicians with relevant expertise.

As part of the appraisal process, MSAC invites input on applications from a range of stakeholders, through both targeted and public consultation. From 1 July 2021, improvements were made to the MSAC consultation process including earlier publication of meeting agendas, ensuring stakeholders were provided with additional time to review applications and provide feedback. Deadlines were also introduced for consultation feedback to be submitted for consideration by MSAC and its sub-committees, ensuring they are provided with sufficient time for review.

Targeted consultation involves health professional and consumer organisations identified by the applicant and/or the Department of Health and Aged Care as having a potential interest in the application given their expertise. From time to time targeted consultation may also occur with an individual who has specialist knowledge or expertise that can inform MSAC's deliberations. The Department of Health and Aged Care, HTA Groups, MSAC and its sub-committees may also seek further advice from clinical experts.

Managed Access Program for medical devices

The Australian Government accepts this part of the recommendation in principle. The Prescribed List (formerly the Prostheses List) reforms continue to develop, aiming to

[59] consultations.health.gov.au/technology-assessment-access-division/msac-proposal-for-a-cost-recovered-pathway-for-cer/

reduce the cost of medical devices used in the private health sector, and improve transparency and efficiency of the application assessment process and the ongoing administration of the Prescribed List. The Department of Health and Aged Care will be considering the appropriateness and applicability of introducing the Managed Access Program for high-cost and high-utilisation medical devices as part of the Prescribed List reforms and improving access to novel technologies.

Parallel TGA and MSAC processing

The Australian Government accepts this part of the recommendation. Applications can already be made to MSAC and TGA under parallel processing arrangements. An application to MSAC can be lodged before relevant therapeutic goods are included on the ARTG, as long as there is evidence the relevant sponsor/manufacturer has commenced the TGA process. As with the parallel process arrangements between the TGA and the PBAC, confirmation of TGA approval (i.e. inclusion of a product on the ARTG) is required before MSAC can finalise its own appraisal of the corresponding medical service/technology. The Department of Health and Aged Care will provide further information on its website to ensure sponsors/manufacturers are aware of parallel processing arrangements.

It is important to note, however, that many HTA applications to MSAC focus on broader services and technologies rather than on the evaluation of products from individual sponsors. In addition, some applicants may only seek regulatory approval and not reimbursement for a product, or alternatively reimbursement only for a service that is not related to regulatory approval of a specific product.

Sponsor presentations to MSAC and pre-submission meetings

The Australian Government accepts this part of the recommendation. MSAC continues to provide opportunities for applicants to present at its meetings (a 'sponsor hearing'). This is available for applications that meet two or more of the following criteria and where the MSAC Executive approves the request:

- The application is requesting public funding for a service/technology that MSAC or its sub-committees view as highly specialised or would be disruptive to the healthcare system.
- Significant issues (clinical or financial) have been raised by MSAC, its sub-committees or the Department of Health and Aged Care during the evaluation of the application.
- Public funding of the requested service/technology would likely have significant patient benefit.
- MSAC has initiated the hearing.

The Department of Health and Aged Care will ensure further information is available on its website about opportunities for pre-submission meetings. The Department of Health

and Aged Care will also review the criteria and procedure for applicants requesting a hearing and seek agreement to any proposed changes from the MSAC Executive.

MSAC's PICO Advisory Sub-committee also allows applicants and their clinical experts to attend its meeting to discuss the proposed medical service/technology.

Pre-submission meetings continue to be available to applicants, and information on how to engage in these meetings was published on the MSAC website in June 2021. The Department of Health and Aged Care will continue to develop a more formal pre-submission meeting process for potential applicants, recognising the wide range of enquiries received.

International collaboration

The Australian Government accepts this part of the recommendation. The Australian Government and the MSAC will explore opportunities for appropriate international collaboration with similar HTA agencies. As each appropriate opportunity emerges, affected applicants will need to be appropriately engaged.

Inclusion of MSAC in HTA Policy and Methods Review

The Australian Government accepts this part of the recommendation in principle. The HTA methods used by MSAC were reviewed and the updated *Guidelines for preparing assessments for the Medical Services Advisory Committee* (MSAC Guidelines) were published in June 2021.[60] The MSAC Guidelines provide advice on the HTA methods that are used throughout the MSAC assessment pathway. The MSAC Guidelines promote processes to ensure Australians have access to safe, clinically effective and cost effective medical services, health technologies and health programs.

The review looked at the Therapeutic Guidelines (version 2.0, March 2016) and Investigative Guidelines (version 3.0, July 2017), and addressed technical method issues raised by MSAC and stakeholders since the last substantial revision. There is now only one set of technical guidelines, which combines Therapeutic and Investigative Guidelines. Public and targeted consultation took place on the Draft MSAC Guidelines throughout 2020 and early 2021 by individuals and organisations. This input was taken into consideration in finalising the 2021 MSAC Guidelines.

The Strategic Agreement does not limit the matters to be considered in the HTA Review. The Reference Committee will consider policy and methods used by the MSAC, focussing on those used for evaluating cell and gene technologies and medicines that are improved by the use of a diagnostic test.

Calendar of meetings and outcomes

The Australian Government accepts this part of the recommendation. The published MSAC calendar currently includes the full timeline up to, and including, the MSAC meeting dates. This includes dates for agenda publication for meetings of MSAC and its sub-committees, application lodgement, provision of documents such as draft PICO

[60] www.msac.gov.au/internet/msac/publishing.nsf/Content/MSAC-Guidelines

confirmations, commentaries and reports of the MSAC ESC to applicants, and public consultation. The Department of Health and Aged Care proposes including fixed post-MSAC timelines for provision of minutes to applicants to enable release of Public Summary Documents as soon as possible on the MSAC website to provide certainty in timeframes for applicants and the public. The Australian Government will consider how to streamline post-MSAC timeframes, including MBS listing timeframes. Streamlining will be facilitated by implementation of processes for standardised redactions to enhance efficiency, transparency and consistency of information published in the Public Summary Documents.

Guidance for sponsors of digital health technologies

The Australian Government accepts this part of the recommendation in principle. As noted above, the HTA methods used by MSAC were reviewed, and updated MSAC Guidelines were published in June 2021. The 2021 MSAC Guidelines include world-leading guidance on digital health technologies linked with investigative technologies (pathology or imaging), where two main types exist: fixed algorithms or self-learning algorithms (also called AI).

However, digital health technologies cover a wide range of technologies, including many which do not relate to a medical service (e.g. digital technologies which allow people to self-manage a condition) and therefore are not linked to public funding arrangements within MSAC's scope for advice. As digital technologies related to medical services evolve, MSAC's guidance will continue to be updated as required, including drawing on the expertise of the Australian Digital Health Agency where relevant.

Benchmarking for MSAC assessments

The Australian Government accepts this part of the recommendation in principle. The Department of Health and Aged Care is continuing to streamline and digitise the processes to support the public funding of health technologies through the progressive roll out of the Health Products Portal to manage applications considered by the MSAC. Alongside this work, the Department of Health and Aged Care is developing a metrics framework to measure and report on MSAC assessments.

Interpretation of responses

Accept	The Australian Government accepts this recommendation, and relevant details regarding implementation are included in the response.
Accept in part	The Australian Government accepts parts of the recommendation, but does not accept other parts.
Accept in principle	The Australian Government generally supports the recommendation or its intent. Some of the details of the recommendation may not be supported and these reasons are outlined in the response.
Note	The Australian Government considers no specific action or response is necessary for this recommendation.

Acronyms and abbreviations

ACSQHC	Australian Commission on Safety and Quality in Health Care
AI	Artificial Intelligence
AMWG	Access to Medicines Working Group
ARTG	Australian Register of Therapeutic Goods
CEEU	Consumer Evidence and Engagement Unit
CIRS	Centre for Innovation in Regulatory Science
CMO	Chief Medical Officer
Compendium	National Teletrials Compendium
CTN	Clinical Trial Network
ESC	Evaluation Sub-committee
FDA	Food and Drug Administration
GBMA	Generic and Biosimilar Medicines Association
GCP	Good Clinical Practice
Gene Technology Act	*Gene Technology Act 2000*
GMO	genetically modified organism
HECS	Higher Education Contribution Scheme
HREC	Human Research Ethics Committee
HTA	health technology assessment
HTA Review	Health Technology Assessment Policy and Methods Review
ICH	International Council for Harmonisation
IGPRG	Inter-Governmental Policy Reform Group
IP	intellectual property
IVD	in vitro diagnostic medical device
LSDP	Life Saving Drugs Program
MBS	Medicare Benefits Schedule
MRFF	Medical Research Future Fund

MSAC	Medical Services Advisory Committee
MSAC Guidelines	Guidelines for preparing assessments for the Medical Services Advisory Committee
National Health Act	*National Health Act 1953*
National Statement	National Statement on Ethical Conduct in Human Research
NBA	National Blood Authority
NBS	Newborn Bloodspot Screening
NBS Framework	Newborn Bloodspot Screening National Policy Framework
NCRI	National Critical Research Infrastructure
NDHS	National Digital Health Strategy
NHMRC	National Health and Medical Research Council
NHRA	National Health Reform Agreement
NICE	National Institute for Health and Care Excellence
NIP	National Immunisation Program
NRF	National Reconstruction Fund
OGTR	Office of the Gene Technology Regulator
Patents Review	Pharmaceutical Patents Review
PBAC	Pharmaceutical Benefits Advisory Committee
PBS	Pharmaceutical Benefits Scheme
PC	Productivity Commission
PICO	Population, Intervention, Comparator and Outcome
Prescribed List	Prescribed List of Medical Devices and Human Tissues Products (formerly the Prostheses List)
PRO	Patient Reported Outcomes
R&D	Research and Development
Rare Diseases Action Plan	National Strategic Action Plan for Rare Diseases
RCRDUN	Rare Cancers, Rare Diseases and Unmet Need
RWE	Real-World Evidence
SSSI	Serious Scarcity Substitution Instrument
TGA	Therapeutic Goods Administration
Therapeutic Goods Act	*Therapeutic Goods Act 1989*
US	United States