



Australian HTA Review

Consultation 1: Report



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Disclaimer

This work was prepared by CHERE during the time period from June to September 2023. The sources used to inform this work were based on submissions to the HTA Review from interested stakeholders. It is possible that there is inconsistency between the sources that were used, and some information may have been overlooked or misrepresented. Notes for three stakeholder forums were completed by the Department of Health and Aged Care and have been included as provided.

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Accronyms

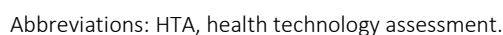
ABF	activity based funding
ACCC	Australian Competition and Consumer Commission
AE	adverse event
AHMAC	Australian Health Ministers' Advisory Council
AI	artificial intelligence
ATAGI	Australian Technical Advisory Group on Immunisation
AU	Australia
CADTH	Canadian Agency for Drugs and Technologies in Health
CAR-T	chimeric antigen receptor T-cell therapies
CIC	commercial-in-confidence
COVID-19	Coronavirus disease 2019
DoHAC	Department of Health and Aged Care
EMA	European Medicines Agency
ES	Executive Summary
ESC	Economic Sun-Committee (Australia)
EMR	electronic medical record
HCP	health care practitioner
HPP	Health Products Portal
HST	highly specialised therapies
HTA	health technology assessment
ICER	incremental cost-effectiveness ratio
IHACPA	Independent Hospital and Aged Care Pricing Authority
ISPOR	International Society of Pharmacoeconomics and Outcomes Research
JBC	Jurisdictional Blood Committee
LSDP	Life Saving Drugs Program (Australia)
MAP	Managed Access Program
MBS	Medicare Benefits Schedule (Australia)
MOA	mechanism of action
MSAC	Medical Services Advisory Committee (Australia)
NACCHO	National Aboriginal Community Controlled Health Organisation
NBA	National Blood Authority (Australia)
NCRAS	National Cancer Registration and Analysis Service (UK)
NHRA	National Health Reform Agreement
NHS	National Health Service (UK)
NICE	National Institute for Health and Care Excellence (UK)
NIHR	National Institute for Health and Care Research
NIP	National Immunisation Program
NSW	New South Wales
NZ	New Zealand
OCS	oral corticosteroids
OECD	Organization for Economic Cooperation and Development
OOP	out of pocket
OTC	over the counter
PBAC	Pharmaceutical Benefits Advisory Committee (Australia)
PBS	Pharmaceutical Benefits Scheme (Australia)
PICO	population-intervention-comparator-outcome
PL	Prescribed List
PROMs	patient-reported outcome measures

PREMs	patient-reported experience measures
PSD	public summary document
QALY	quality adjusted life year
QoL	quality of life
RC	Reference Committee
RCT	randomised controlled trials
RSA	risk sharing agreement
RWE	real world evidence
SA	South Australia
SBU	Swedish Agency for Health Technology Assessment and Assessment of Social Services
SID	supplier induced demand
SMA	spinal muscular atrophy
SMC	Scottish Medicines Consortium
SPA	shared pricing agreement
SROI	social return on investment
TGA	Therapeutic Goods Administration (Australia)
TLV	Dental and Pharmaceutical Benefits Agency (Sweden)
UK	United Kingdom
WTP	willingness to pay

A total of 106 stakeholders lodged written submissions to Consultation 1 of the Health Technology Assessment (HTA) Review. In addition, the HTA Reference Committee hosted three separate consultation forums which involved five patient/community organisations and two industry organisations. This report presents a qualitative synthesis of those submissions and proceedings from those forums (input from written submissions and forums is collectively referred to as consultation submissions).

However, consultation submissions highlighted a number of main barriers to timely access to care: delays in access to care, in particular the time from registration to funded access for drugs was considered to be overly long relative to other comparable countries; delays in (or no) access for care for those with rare/orphan conditions; a system that is overly focused on randomised controlled trials and the 'economic value'; and an absence of harmonised HTA across the Australian health care system.

ES Figure 1: Emergent themes from stakeholder submissions



Stakeholders provided a large number of options for change which focused on implementing alternative approaches for subsidising technologies (including subsidising some/all technologies at the time of registration, with HTA assessment to follow); expanding the scope of evidence considered to give more weight to the inclusion of observational data (real-world evidence); incorporating broader aspects of value, in particular patient reported experience measures and patient reported outcome measures in assessments of technologies; facilitating greater community participation HTA; moving away from a one-size-fits all approach to HTA to recognise specific technologies and indications; and refining the overarching HTA processes to better align across agencies, and allow for non-sponsor initiated submissions.

2 Health Technology Assessment Policy and Methods Review

As part of the ongoing HTA Review (2023), the Department of Health and Aged Care conducted a period of open consultation (Consultation 1: 8 weeks ending 6 June 2023) during which it sought submissions to the Review from interested stakeholders. In addition, it invited interested stakeholders to participate in open stakeholder forums with the HTA Reference Committee (RC). This document outlines the approach to the analysis of the written and verbal (forum based) input to those consultations (input from written submissions and forums is collectively referred to as consultation submissions). Overall, the analysis in this document provides a thematic summary of the information presented without interpretation, fact-checking or rebuttal of the input in the consultation submissions.

The questions included in the HTA consultation (see Appendix 1) were structured according to seven key areas of interest:

1. Elements and features that are working effectively
2. Current or future barriers to earliest possible access
3. Current or future barriers to equitable access
4. Elements and features that detract from person centredness
5. Perverse incentives
6. Areas of interest
7. Other

There were nine core questions (Table 1), with various sub-questions seeking further detail (examples to illustrate the points being made), across those seven areas.

Table 1: Consultation questions

Area of Interest	Consultation Questions
Elements and features that are working effectively	<ol style="list-style-type: none">1. Are there any elements and features of HTA policy and methods in Australia that are working effectively?<ol style="list-style-type: none">a. Are you able to provide detail of any elements and features of HTA policy and methods that are working effectively? Please use specific details where possible.b. Are you able to provide details of positive outcomes resulting from Australia's HTA policies and methods? Please use specific examples where possible.
Current or future barriers to earliest possible access	<ol style="list-style-type: none">2. What are the elements and features of HTA policy and methods that are acting as a current barrier to earliest possible access?3. What are the elements and features of HTA policy and methods that may act as a future barrier to earliest possible access?4. Would you like to provide feasible options or suggestions you have to improve elements of HTA policy and methods that are acting as a current or future barrier to earliest possible access?

Area of Interest	Consultation Questions
Current or future barriers to equitable access	<p>5. What are the elements and features of HTA policy and methods that are acting as a current or future barrier to equitable access?</p> <p>a. Are you able to provide details of feasible options / suggestions to improve elements of HTA policy and methods that are acting as a current or future barrier to equitable access?</p>
Elements and features that detract from person centredness	<p>6. Are you able to provide details of any elements and features of HTA policy and methods that may be detracting from person- centeredness?</p> <p>a. Are you able to provide details of feasible options / suggestions to improve elements of HTA policy and methods that are detracting from person-centeredness?</p>
Perverse incentives	<p>7. Are you able to provide details of elements of features of HTA policy and methods that are causing or could cause unintended consequence or perverse incentives?</p> <p>a. Are you able to provide details of feasible options / suggestions to improve elements of HTA policy and methods that are creating unintended outcomes or perverse incentives either currently or in the future?</p>
Areas of interest	<p>8. Are there any HTA or reimbursement models, or elements thereof, utilised in other countries that you believe should be considered for potential adoption in Australia, or that it would be good for the Reference Committee to understand?</p>
Other	<p>9. Is there any other information relevant to the Review not provided above that you would like to add?</p>

Abbreviations: HTA, health technology assessment.

3 Methods

3.1 Extraction and coding

The analysis of the consultation submissions was conducted by summarising the responses to each question (as identified in Table 1) by stakeholder group. Themes arising from the consultation submissions were identified via a combined deductive/inductive approach. Consultation inputs were reviewed against the a-priori codes in Table 2 to identify relevant excerpts (deductive). This was supplemented by an inductive approach to identify emergent themes not captured by the a-priori codes (which recognises that the a-priori codes are not exhaustive in nature). Themes were consolidated across stakeholder groups into an agreed analytical framework by applying the resulting codes.

Table 2: A-priori codes

Code	Scope/definition (submission refers to:)
Evidence requirements (clinical/cost-effectiveness)	<p>The evidence required to demonstrate that a health technology is effective, safe and cost-effective (value for money). Statements may be framed as barriers/facilitators faced within the existing HTA system.</p> <p>The use of 'real-world evidence' (observational data) to meet evidentiary requirements.</p>
Methods of assessment	<p>The methods (analytical techniques) used to demonstrate that a health technology is effective, safe and cost-effective (value for money).</p> <p>Emerging analytical techniques not applied in the HTA in Australia that are considered to be of benefit.</p>

Code	Scope/definition (submission refers to:)
Timeliness	The desire for access to new (funded) health technologies as early as possible. Steps that may be taken to enhance the speed with which decisions are made and health technologies are made available on a funded basis.
Equity	The potential for the HTA system to affect (positively or negatively) equity of access to health care.
Consumer/Community Engagement	The desire to achieve a patient-centred approach to HTA. The inclusion of consumer/community values within the HTA system and resulting decision-making.
Transparency	Information of how decisions are made by HTA agencies, the factors considered and how they might be combined in reaching decisions. Information on the explicit steps required to achieve a recommendation for subsidy from a HTA agency.
Managing uncertainty	The steps that may be taken through the reimbursement process to address the uncertainty associated with clinical evidence or evidence of cost-effectiveness. The potential to provide data on an ongoing basis, post-reimbursement, to address uncertainty associated with the initial decision (coverage with evidence development). Linking payments for health technologies to the outcomes observed in clinical practice (outcome-based-payments).
Special pathways	Consider specific funds for specific indications (e.g., cancer drug fund) Consider specific decision criteria or weights for specific indications (e.g., rare diseases) or population groups (e.g., First Nation peoples)
International benchmarking	International practices in HTA considered to be desirable for inclusion in Australia. HTA practices/processes where Australia is considered to be a leader. International practices in HTA Australia should not consider adopting.
Emerging technologies	New health technologies for which a new approach to HTA is considered desirable (or for which the current system is considered not fit for purpose).

Abbreviations: HTA, health technology assessment.

Options for change (which may have been framed within the consultation submissions as recommendations, suggestions or statements on how a 'future' HTA system could operate) provided within the consultation submissions were extracted and summarised by stakeholder group. These were subsequently reported against the themes to which they contribute and categorised by the key topic to which they pertain (e.g., reforming timelines). The focus in the body of the report are those options for change which provided some detail on how the change might be implemented (all options for change are included in the relevant appendix).

3.2 Reporting

The findings of the analysis are reported in three parts:

- a high-level summary of the key inputs giving rise to the emergent themes across all questions.
- a narrative summary of the themes, noting similarity or divergence of themes across sectors.
- a summary of the options for change, reported by theme.

Throughout the report, quotes are used to illustrate themes in terms of: endorsement of a theme; and, divergent views within a theme. Unique inputs, particularly those proposing significant changes/additions to existing processes or that relate to specific novel technologies are also highlighted.

Where a consultation submission has referred to previous submissions to other processes (such as the House of Representatives Inquiry into approval processes for new drugs and novel therapeutics), reference to that submission (and the specific aspect of the previous submission cited) is cited. No additional information from those previous submissions has been extracted (on the basis that including such additional information may misrepresent the intent of consultation submissions to the HTA Review).

4 Results

4.1 Consultation materials

There were 113 submissions received as part of the consultation (Table 3) with patient and consumer organisations (35%) and pharmaceutical/medical technology companies (33%) comprising nearly two thirds of those making submissions (Table 4). In many instances, stakeholders lodged more than one document to the consultation process, comprising input to the online questions along with a supporting attachment. Stakeholders contributing to the Review are listed in Appendix 2 (this includes stakeholders who indicated that their submissions were not for publication and those participating in the forums).

Table 3: Consultation submissions - method of participation

Option	Number of submissions (%)
Consultation hub only - written document	75 (66.4)
Email only - written document	23 (20.4)
Consultation hub and email - written document	8 (7.1)
Forum participation (number of organisations)	7 (6.2)
Total	113

Table 4: Stakeholder submissions

Stakeholder group	Total (%)	Documents	Options for change
Patient or consumer organisation (Patient)	39 (34.5)	40	43
Pharmaceutical / medical technology company/ Industry associations (Industry)	37 (32.7)	56	100
Research & Consultancy (Research)	14 (12.4)	20	99
Peak body (Peak)	9 (8.0)	15	45
State and Territory Governments (Jurisdiction)	6 (5.3)	10	17
Other (Others)	8 (7.1)	11	30
Total	113	152	334

Note: labels shown in parentheses after each stakeholder category were used to differentiate specific quotes used in this report.

The count under 'Options for change' refers to the total number of options extracted per stakeholder group. Options may have been repeated within and across stakeholder groups.

Stakeholder groups have been grouped based on self-selection by those making submissions (see Appendix 2 for composition of each category).

4.2 Extraction of Materials

The summarised extracts of the consultation submissions, by consultation question and stakeholder group, are presented in Appendix 3. In addition, summaries from the stakeholder forums are provided in Appendix 4. The key elements from all these inputs by consultation question, as informing the subsequent emergent themes are presented in the following subsections.

4.2.1 Elements and features that are working effectively

Inputs from the stakeholder submissions in response to which elements and features of the HTA system are working effectively are summarised in Table 5. Key elements common across stakeholder groups include:

1. Flexibility in approach and decision-making. Stakeholders across groups reported that the absence of an explicit incremental cost-effectiveness ratio (ICER) threshold for decision-making allows the current HTA process to be flexible in its assessment of evidence. Some stakeholders noted the process, particularly with respect to the Pharmaceutical Benefits Advisory Committee (PBAC) has shown itself to be flexible in being able to accommodate new technologies, resulting in Australian patients having access to care.

".. there is flexibility to work with threshold values for the incremental cost-effectiveness ratio (ICER), to make some landmark approvals for truly innovative therapies to treat cystic fibrosis, spinal muscular atrophy and other rare diseases" (Peak).

"For example, the lack of a specified ICER threshold allows some flexibility in PBAC decision-making, meaning that the PBAC can incorporate less quantifiable factors such as high clinical need or the rarity of a condition into deliberations about the cost-effectiveness of a medicine" (Industry).

2. A robust, expert process, supported by independent review. Stakeholders commented that a key strength of the existing process for HTA in Australia is the rigour applied to the assessment of the evidence, that the process is undertaken by multidisciplinary committees of experts and is supported by independent review of the evidence.

"... the work of the Therapeutics Goods Agency (TGA), Pharmaceutical Benefits Advisory Committee (PBAC), Medical Services Advisory Committee (MSAC), the National Immunisation Program (NIP), and the Jurisdictional Blood Committee (JBC), which oversees the national blood arrangements that are managed by the National Blood Authority (NBA). Each of these entities has robust mechanisms to ensure that medical devices, treatments, therapies, pharmaceuticals, and immunisations are rigorously assessed to ensure patient safety, clinical effectiveness, and cost-effectiveness for the healthcare system." (Jurisdiction)

3. Engagement of consumers and the public. Stakeholders also commented on the strength within the existing system associated with the process for community and consumer input. This was seen as an important element of system transparency but also in ensuring that the evidence assessed, and process for HTA, reflect community values.

".. is supportive of the HTA Consumer Evidence and Engagement Unit; this consumer-focused initiative and future initiatives that will help make it easier for consumers to play a more active role and be more valued in the HTA process. ... acknowledges and supports an increasing emphasis on the elevation of the patient voice and inputs to health system reforms including careful consideration of how it can further improve HTA policy and methods. It is also important to acknowledge that most of the new melanoma treatments have been listed on the PBS and are available to patients that need them currently and that Australia has a relatively good position in comparison to many other countries around the world " (Patient)

Table 5: Elements and features working effectively

Stakeholder group	Summary of key inputs extracted
Patient	Consumer input (via evidence, representative, committees, online hub, public consultation); Publicly available high-quality evidence; Transparent process for common disease; Flexibility to deal with other conditions (including specific programs); First Nations representation and focus.
Industry	Flexibility in decision-making (no set ICER); Confidential pricing; Capacity to consult with DoHAC and PBAC (including post-decisions); Parallel processing; Re-entry pathways.
Research	Detailed & timely evaluations; Independent expert advice; High evidentiary standards; Publicly available reports (transparent); Flexibility in decision-making.
Peak	Rigorous process, expert committees; Consumer Evidence and Engagement Unit & consultation hub effective at 'upskilling' at facilitating input; Expedited pathways reduced resubmission times.
Jurisdictions	Strong support for expert, detailed HTA; Independent multidisciplinary committees; Engagement with the committees.
Other	PBAC structure works well for simple single non-complex drug approvals; Value for money; MSAC streamlined approvals process; Project Orbis (registration); Stakeholder input; Positive outcomes for consumers

Abbreviations: DoHAC, Department of Health and Aged Care; HTA, health technology assessment; ICER, incremental cost-effectiveness ratio; MSAC, Medical Services Advisory Committee; PBAC, Pharmaceutical Benefits Advisory Committee

4.2.2 Current or future barriers to earliest possible access

Inputs from stakeholder submissions in terms of identifying current or future barriers to access are summarised in Table 6. Key elements common across stakeholder groups include:

1. Patient access gap (delays in timely decisions). A commonly reported barrier across stakeholder groups was the perceived delay associated with access to reimbursement technologies, notably drug therapies. This delay was couched in terms of the time from registration (on the Australian Register of Therapeutic Goods) to being made available as a reimbursed item on the Pharmaceutical Benefits Scheme (PBS). Numerous stakeholders pointed to Germany, France and Japan as comparable overseas markets where there is no apparent delay between the time at which a product is registered and is reimbursed. This sentiment was best summarised by the following statements from the Medicines Australia submission:

"An analysis of PBAC recommendations, between March 2021 and March 2022, leading to PBS listing indicated that 'ever' cost-effective submissions (where cost-effectiveness was utilised in at least one of the submissions leading to the listing) had a mean patient access gap of approximately 600 days. Updating this analysis to include PBAC recommendations until March 2023 (including listings as of 6 May 2023) results in a similar time to listing of 603 days. For listings based on cost-minimisation (typically

implying no incremental cost to Government) the mean patient access gap was 408 days.

and

For those innovative, first indication medicines that are funded in Australia the average time from local regulatory registration to public funding was most recently reported as 466 days, much longer than other OECD countries such as Germany, France, Japan, the UK, Switzerland, Norway, Sweden, Finland and Austria."¹²

2. Overly focused on randomised controlled trials (RCT). Stakeholders commented that the current process is overly focused on the primacy of evidence from RCTs. This is particularly problematic in that it: downplays the potentially important role that 'real-world evidence' (observational data) may have in HTA; may disadvantage those indications (e.g., rare diseases) or technologies for which there are small patient numbers meaning that it is not possible to conduct RCTs; or reduce the importance of qualitative evidence, often from the patient and societal perspective, as part of HTA.

"Prioritisation of evidence from randomised controlled trials compromises (sic) a barrier to timely, equitable access to rare disease health technologies; in some cases, this may discourage companies from submitting therapies for particular indications (e.g., The sponsor of Miglustat for patients with Niemann-Pick Type C has decided not to pursue reimbursement via PBAC/LSDP due to a lack of evidence, creating uncertainty for patients currently accessing this therapy via hospital funding/compassionate access)."
(Peak)

¹ This quote from the Medicines Australia submission to the Review cites metrics on time to listing from Millar, D, Commercial Eyes Analysis. Presentation by Douglas Millar at ARCS 2022 Conference, ARCS, Australia; 2022 (cited as reference 8 in the Medicines Australia submission); and Medicines Australia. Medicines Matter: Australia's Access to Medicines 2016-2021. Australia: Medicines Australia; 2022. Available from: <https://www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2023/04/Medicines-Matter-2022-FINAL.pdf> (cited as reference 6 and 7 in the Medicines Australia submission).

² For additional information on time to a positive decision for medicine coverage in OECD countries see: Chapman, S., A. Szklanowska and R. Lopert (2023), "Exploring the feasibility of monitoring access to novel medicines: A pilot study in EU Member States", OECD Health Working Papers, No. 151, OECD Publishing, Paris, <https://doi.org/10.1787/8c1d16c4-en>; and Chapman, S., V. Paris and R. Lopert (2020), "Challenges in access to oncology medicines: Policies and practices across the OECD and the EU", OECD Health Working Papers, No. 123, OECD Publishing, Paris, <https://doi.org/10.1787/4b2e9cb9-en>.

"Incorporating real-world evidence (RWE) into the HTA process can provide valuable insights into the safety, effectiveness, and economic impact of treatments in real-world settings. By analysing RWE alongside traditional clinical trial data, the HTA system can better assess the long-term benefits and risks of innovative technologies. This approach helps address uncertainties that may exist in controlled clinical trial settings and provides a more comprehensive understanding of treatment outcomes in diverse patient populations." (Research)

3. Overly focused on the ICER/economic value. Consistent with the inputs on the focus on the nature of evidence, stakeholders stated that the current focus on the ICER and costs of therapies (e.g., individual therapy costs and total cost to Government) were barriers to access in that they ignored other potential sources of value. In particular, the focus on the ICER - with its metric of the cost per quality adjusted life year (QALY) gained - was seen to exclude important patient focused values and indirect benefits (such as return to work, return to education and other elements of potential value that might be captured in a social return on investment analysis).

"The HTA system's narrow definition of value and impact poses another barrier to timely access. Traditional measures, such as cost-effectiveness and clinical efficacy, often take precedence, disregarding broader aspects of patient well-being and societal impact. By expanding the evaluation criteria to include patient-reported outcomes, quality of life improvements, and the broader socio-economic benefits of innovative therapies, the HTA system can better reflect the true value and potential impact of these interventions, allowing for more efficient access." (Research)

4. Specific challenges with respect to methods. There were four specific areas of methodology noted variously by a number of stakeholders, largely within the Industry stakeholder group, as challenges leading to access barriers:
 - a. the current recommended discount rate for HTA in Australia (5%) was considered to be too high relative to international comparisons (1.5% in the UK) which disadvantaged some indications, particularly those in the prevention setting;
 - b. the choice of comparator as based on the lowest cost-alternative does not reflect the choice of therapy likely to be replaced most in practice and disadvantages new, innovative products;

- c. there is often disagreement in the PBAC process on the population-intervention-comparator-outcome (PICO) statement; and
- d. indirect comparisons in PBAC do not follow world best-practice with respect to the use of network-meta-analysis.

"Adopt the methodology for indirect comparisons accepted in other HTA markets (e.g., NICE) for the purpose of demonstrating clinical superiority and cost-effectiveness."
(Industry)

"Reduce the base-case discount rate for PBAC evaluations to 1.5% in line with comparable best practice HTA countries." (Industry)

"The original intent of the PBAC Guidelines was for the comparator to be the medicine(s) most likely to be replaced in clinical practice. As demonstrated, the requirement for new medicines to be price referenced to the lowest cost comparator has, in some cases, resulted in novel medicines being delayed or even not launched in Australia. This issue could be overcome by allowing price referencing to the medicines most likely to be replaced as per the Guidelines, or for a weighted price to be applied in cases where there is more than one appropriate comparator with different prices." (Industry)

5. Cost of submissions. Stakeholders noted that in addition to the evidence, methods and assessment processes, the cost associated with developing and lodging applications was a disincentive, particularly for those technologies where there was a low likelihood of reimbursement or there was likely to be a small, reimbursed indication (e.g., rare diseases).

"Considering that it typically takes several submissions to achieve a PBS listing – if a successful outcome is ever achieved -- companies need to budget almost \$2M per indication for a single drug. If there are further indications in the pipeline these must also be budgeted – potentially costing another \$2M per indication." (Industry)

Table 6: Current or future barriers to access

Stakeholder group	Summary of key inputs extracted
Patient	Delays in PBAC decisions resulting in resubmissions; Lower value placed on non-RCT evidence; Lengthy price negotiation; Rigid policies with respect to new medicines/technologies; PBAC & MSAC not in synch; Not responsive to medicines for rare disease (poor industry incentives); Specific First Nations issues not met (lack of priority meds, issue of affordability, consultation).

Industry	Timelines – delays in listing (long process; high opportunity cost); Overly focused on ICER (WTP low) absent RWE and societal values; Misalignment of HTA for drugs, devices and services; Lack of transparency on decision-making; Methods challenges (data, comparator, discount rate).
Research	Rigid process (timeframe & lack of engagement); Overly focused on RCTs (absent RWE, patient value); Complex & substantial time delays (reg to reimbursement); Not fit for purpose for rare disease, gene therapy, genomics; Overly focused on ICER as value; Methods challenges (PICO, comparator, discount rate).
Peak	Lack of coordination between govt levels; Acute care sector largely absent (from evidence or implementation); Focus on RCTs is barrier to timely access (esp. rare disease); Cost of multiple submissions; 2-step LSDP process; Lack of horizon scanning.
Jurisdictions	Approval based on early data is risky; CIC limits review and transparency; PBS restrictions conflict with clinical guidelines; PBAC & MSAC not in sync.
Other	Cost of submission; Regulatory and reimbursement barriers for repurposed medicines; Timeliness - delays in processing, listing and reporting; No horizon scanning; Tight restrictions; Not fit for purpose (e.g., digital health); Methods - comparator; Need greater expert opinion at all stages; Lack of clarity.

Abbreviations: CIC, commercial in confidence; HTA, health technology assessment; ICER, incremental cost-effectiveness ratio; LSDP, Life Saving Drugs Program; MSAC, Medical Services Advisory Committee; PBAC, Pharmaceutical Benefits Advisory Committee; RCT, randomised controlled trial; RWE, real-world evidence; WTP, willingness to pay.

4.2.3 Current or future barriers to equitable access

Stakeholders raised a number of barriers in common with respect to the impact on equitable access:

1. Commercial disincentives to seek reimbursement for technologies for rare diseases. A particular barrier to equitable access was noted with respect to the lack of incentives in the system currently for sponsors to lodge applications for the reimbursement of technologies for rare diseases. These disincentives were seen to arise due to: (1) the lower levels of evidence (not RCT) typically associated with rare diseases - this evidence was considered to be less likely to be positively received as part of the HTA process; (2) high, often up-front, treatment costs resulting in high ICERs; (3) high costs of submitting applications for reimbursement.

"When evaluating rare diseases, the PBAC applies the same process to applications treating a small number of patients (e.g., 5 patients) as it does to applications treating a larger number of patients (e.g., 5,000 patients). Currently, there is a specific pathway in place for ultra-rare conditions through the Life Saving Drugs Program (LSDP). However, for rare conditions that do not meet the LSDP criteria, there is little flexibility in the process. As a result, Sponsors may be discouraged from seeking reimbursement for treatments for rare conditions in Australia with resultant challenges for patients who may have limited treatment options available to them". (Industry)

2. Lack of integration of HTA across systems leading to higher costs and 'postcode lottery for access'. Consultation submissions, particularly those from the Jurisdictions, highlighted that

the complexity of funding and providing health care in Australia is another potential barrier to equitable access. This was highlighted with particular reference to those therapies that might be funded by the Commonwealth (i.e., via MSAC or PBAC) but where delivery is via the jurisdictions, and therefore access may differ on a hospital-by-hospital basis. Most often, reference was made to potential barriers arising with respect to access to highly specialised therapies (HSTs) such as CAR-T, but other stakeholders noted that inequities might also arise where some hospitals choose to fund access to drugs rejected by the PBAC (and access to those same drugs might be denied by other hospitals).

"The National Health Reform Agreement 2020-25 only applies to medicines where the annual treatment cost at the commencement of funding exceeds \$200,000 per patient.³ However, medicines with a price below this threshold but which represent a high cost at the state level (i.e., above \$300,000 to \$450,000 for SA Health) are not included in this agreement. In these situations, local decision makers must determine whether they will fund these medicines. These decisions can be made at the state, district or hospital level and because this decision-making process is not part of a national process or policy framework, different states, health networks and hospitals may make different decisions with regard to the same high cost medicine. This results in inequity of access to these medicines across Australia." (Jurisdiction)

"There is no support with post-MSAC approval education of medical professionals, which likely means that some professionals are not accessing tests that would be beneficial for their patients because they are unaware of their availability or significance to their patient. Further, MSAC's approaches to which medical professional can order tests; the rebate amount, and for genomic tests whether there is gene list, or no gene list are all areas where inequity could be introduced, in terms of patient access, timeliness of result and results reported." (Research)

3. Lack of coordination between regulatory and reimbursement processes. Stakeholders also noted two keyways in which a lack of alignment between regulatory and reimbursement processes affects equity of access. First, with respect to misalignment between registration and reimbursement timing; for some technologies this means that those with the means to

³ For a definition of high cost, highly specialised medicines as applied in the National Health Reform Agreement 2020-25 see: https://federalfinancialrelations.gov.au/sites/federalfinancialrelations.gov.au/files/2021-07/NHRA_2020-25_Addendum_consolidated.pdf

access care (i.e., those who can afford to pay, either privately or via fund raising) can access care sooner via the private market compared with those who do not have the same access to funding. Second, rescheduling of medicines at the regulatory level to make them over the counter may mean that some patients face prohibitive out-of-pocket costs if they are no longer able to access therapies via the PBS (for example).

"Where products are approved for marketing but are yet to gain a PBS approval, it creates a postcode lottery of access, where: - those wealthy enough can access new treatments - those who have access to medication access programs can access new treatments - those who live in jurisdictions that support funding can access new treatments. The Council of Australian Therapeutic Advisory Groups do attempt to improve communications between jurisdictional medication governance groups, in an attempt to minimise this inequity." (Jurisdiction)

"Changes to the Fluticasone Inhaler listing have resulted in in-equitable access for patients, particularly those who are most vulnerable such as remote patients and those of limited means. The change in listing mean the options for treatment for these patients was undesirable including: referral to a specialist which is considerable burden to remote patients and the public health system, or providing access via a private prescription which is costly for those patient of limited means, or prescribing other alternatives with potential negative health impacts in contradiction to Australian asthma guidelines for children under 6 years of age." (Jurisdiction)

Table 7: Current or future barriers to equitable access

Stakeholder group	Summary of key inputs extracted
Patient	Public & private system disparity; OOP costs prohibitive (and can include access to HCP); Regional disparities; Move to OTC negative impacts disadvantaged in terms of co-payment and pharmacist support.
Industry	Commercial barriers to listing for rare diseases; Methods challenges (discount rate, comparator); Focus on ICER (low WTP) – less consideration of severity, burden, indirect costs, equity; Australia behind in terms of access; Risk minimise rather than risk share.
Research	Not responsive to patient needs; Lack of guidance on implementing HTA (MBS); Intergenerational inequity.
Peak	Postcode care (due to lack of integration of HTA); Lack of commercial interest (rare diseases); focus on cost for rare diseases.
Jurisdictions	Risk of postcode funding for some technologies; Disconnect from funding in recommending drugs that are hospital based; Disconnect between regulatory and reimbursement process; Insufficient resource to do HTA.
Other	One size fits all process not applicable to all technologies (precision medicine, digital health); Age limits on PBS listings; Focus on sponsor lead submissions; Lack

Abbreviations: HCP, health care practitioner; HTA, health technology assessment; ICER, incremental cost-effectiveness ratio; MBS, Medicare Benefits Schedule; OOP, out of pocket; OTC, over the counter; PBS, Pharmaceutical Benefits Scheme; WTP, willingness to pay.

4.2.4 Elements and features that detract from person centredness

There were two principal elements commonly raised across the consultation submissions that detract from person centredness (see Table 8 for others):

1. A lack of engagement with consumers. Through their submissions stakeholders noted that while there is positive engagement from consumers in the HTA process in Australia, in general that engagement occurs late in the HTA process, is restricted to specific consumer representatives on the Committees or those well-resourced to engage with the online input process, suffers from a lack of co-design with consumers with respect to the focus and methods of HTA, and does not provide consumers with feedback on the input that they do provide to the process (and hence does not allow for learning and adaptation about what matters with respect to HTA).

"It is positive that there is a pathway for consumers to make submissions to the PBAC in support of applications, to highlight the impacts of access or lack of access to particular medicines and the outcomes that are important to them. However, there is not much information provided about how these submissions are used and how they have impacted on the outcome of the application." (Patient)

2. Overly focused on clinical and economic evidence. In line with the barriers identified with respect to early access, a focus on clinical and economic evidence was identified as hampering patient-centredness. In particular, it was felt that the current focus on RCT driven clinical outputs and the attending measures of ICER were absent patient relevant outcomes as might be captured through the use of patient reported experience measures (PREMs, pertaining to the process and experience of accessing and receiving care) and patient reported outcome measures (PROMs) that might not otherwise be captured via standard measures of quality of life (as captured in assessments of quality of life in estimating QALYs). Several stakeholders identified that this was particularly relevant in the context of gene therapies and genomic medicines where the 'value of knowing' has now been recognised as an important, patient-centred outcome to be incorporated in valuing technologies.

"...current HTA policy and methods are not person-centred because they do not focus on the things that matter most to people and do not routinely measure the impacts of not funding cancer treatments for patients and the costs for children, partners, employers, governments, and communities. Social return on investment (SROI) analyses do not form part of the cost-effectiveness analysis under the current PBAC guidelines. Instead, there is provision for SROI analysis to be submitted as 'other' information, under part 5 of the guidelines, however this provision may be underutilised." (Patient)

"...there seems to be a perception that some HTA assessors tend to consider patient experiences lower in the evidence hierarchy and thus have limited impetus to integrate this type of evidence in the evaluation process. Use of objective tools (patient reported experience measures (PREMs) and patient reported outcome measures (PROMs) to measure patient experience/outcomes should address these potential concerns by HTA bodies. By incorporating PREMs into the evaluation of a medicine, HTA bodies can gain insights into how well the treatment aligns with patients' needs, expectations, and preferences. By including PROMs in HTA assessments, decision-makers can better understand the tangible benefits and potential trade-offs associated with a particular medicine." (Research)

Table 8: Elements and features that detract from person-centredness

Stakeholder group	Summary of key inputs extracted
Patient	Lack of co-design (consultation tokenistic); Overly focused on economic value, not human; No feedback on consumer input; no recognition of 'right-to-try'.
Industry	Lack of engagement/consultation for consumer input (inc. carers); Insufficient consumer/patient representation (Citizens Counsel); One size fits all does not recognise specialist medicines/populations; Does not reflect social values and patient experience; Focus on ICER rather than patient benefit.
Research	Overly focused on clinical evidence/ICER – absent of patient centred values (PREMs/PROMs); Lack of co-design with consumers; Involve consumers throughout HTA process.
Peak	Reimbursement criteria not aligned with clinical practice; RCT data may be limited/not reflect impacts on patients – insufficient weight placed on consumer values; lack of engagement with consumer organisations on evidence required/feedback; 'value of knowing' not applied routinely in HTA.
Jurisdictions	Limited clinical evidence of impact on First Nation peoples.
Other	Limited involvement of patient input, low weighting of patient input and limited transparency/education regarding the process; Move away from individual diseases to 'molecular' approach to technologies; Consumer groups may not be seen equitably (focus is on some diseases, or strong advocacy groups).

Abbreviations: HTA, health technology assessment; ICER, incremental cost-effectiveness ratio; PREMs, patient reported experience measures; PROMs, patient reported outcome measures; RCT, randomised controlled trials.

4.2.5 Perverse incentives

Key inputs arising from stakeholder submissions on perverse incentives in the system are summarised in Table 9. Those arising most commonly across submissions were:

1. Price signalling. Stakeholders noted that the current HTA process, particularly with respect to pharmaceuticals, is such that there is a perception that first-time submissions will always be rejected - largely because the PBAC adopts a conservative approach to dealing with uncertainty. The perception is that sponsors, therefore submit on the basis of a higher price believing that they will be asked for a price reduction; and the PBAC reviews submissions on the basis that the initial price is higher than might otherwise be required.

"The cautious approach adopted by the PBAC towards uncertainty can lead to unintended consequences. During the evaluation process, parameters are often adjusted to reflect conservative estimates, resulting in a reduced economically justifiable price for new medicines. As a result, sponsors are incentivised to submit a higher initial price, anticipating negotiation and multiple resubmissions. This can lead to prolonged timelines for PBS listing, limiting patient access to essential medicines and increasing costs for both sponsors and the government." (Research)

2. Choice of lowest cost alternative as the comparator. Some stakeholders also noted that, in addition to affecting HTA methods, the interpretation of the choice of comparator under the 1984 Health Act, governing the consideration of cost-effectiveness by the PBAC, as being the 'lowest-cost alternative' was a disincentive to sponsors seeking to introduce new, innovative therapies.

"The original intent of the PBAC Guidelines was for the comparator to be the medicine(s) most likely to be replaced in clinical practice. As demonstrated, the requirement for new medicines to be price referenced to the lowest cost comparator has, in some cases, resulted in novel medicines being delayed or even not launched in Australia." (Industry)

3. Funding recommendations in absence of implementation. In alignment with barriers to equitable access, stakeholders noted that the practice of making decisions about funding (e.g., via the MBS or for HST via the National Health Reform Agreement) without corresponding recommendations on implementation had the potential to result in unintended consequences; that is, sponsors may have incentives to seek funding using earlier, more immature data, which

may represent a safety risk. In addition, while some aspects of service provision (e.g., drug supply) might be funded via one aspect of the system (PBS), other aspects of that service (e.g., drug administration) may not be funded in a cost-effective manner in terms of how hospital (e.g., activity based funding) and health care service deliver is funded.

"Early access creates a risk that is ultimately worn by the Australian public financially which can also result in minimising funding for alternative, cost-effective health technologies for different disease states. HTA policy and methods should ensure flexibility to cater to the access of treatment on promising evidence, but also robust to mitigate risk and support clinicians and patients when this does not translate into confirmatory evidence." (Jurisdiction)

Table 9: Key aspects of perverse incentives

Stakeholder group	Summary of key inputs extracted
Patient	Rigid system creates perverse incentive for rare disease (industry does not want to submit applications).
Industry	Price signalling; Least expensive comparator disincentive to innovation; Imbalance in F1 and F2 prices that don't reflect MOA/AE; Conservative stance on uncertainty (adopt risk management/sharing); Fund items (MBS) without regard to access; Compassionate access not 'universal' – not a reason to avoid listing; Information seeking - best advice on a submission comes from committees (lodge submissions to plan for second submission).
Research	Price signalling; Least expensive comparator disincentive to innovation; RSAs shift risk to sponsors, reduce incentive to list.
Peak	Cost-shifting due to PBS not available to public hospital inpatients; fee-for-service (MBS) results in 'SID'.
Jurisdictions	Funding HST early results in sponsors submitting with immature data; HTA pathways for gene technologies unclear; Pipeline agreements challenging; ABF may not support use of cost-effective technology.
Other	Value of innovation and clinical effectiveness need to be recognised to avoid unintended consequence of not listing new therapies that don't fit neatly into the system; Lack of incentive to license two therapies for use in combination, rather than relying on combination therapy across sponsors.

Abbreviations: ABF, activity based funding; AE, adverse event; HST, highly specialised therapy; HTA, health technology assessment; MBS, Medicare Benefits Schedule; MOA, mechanism of action; RSA, risk sharing agreement; SID, supplier induced demand.

4.2.6 Areas of interest

Across stakeholder submissions there were three areas of interest raised most commonly:

1. Horizon scanning. Stakeholders noted that the absence of a dedicated national horizon scanning process limited the capacity of the system to plan for new and emerging technologies, or to consider the introduction of technologies that might not otherwise be put

forward by sponsors. This was perceived as particularly challenging in new and emerging technologies, such as HST, gene therapies and genomic medicines.

"The government also should have a role in being more active in horizon-scanning and needs analysis to identify new and emerging treatments that may not otherwise be presented for HTA through current processes that rely only on sponsors." (Patient).

2. Digital health and artificial intelligence (AI). There was recognition by some stakeholders that the nature of health technologies is changing, particularly with the emergence of digital interventions and the potential for AI to be used as part of health care. The application of HTA methods to these technologies should be investigated.

"The use of technologies, such as smart phones, social networks, and internet applications, is not only changing the way we communicate, but also providing innovative ways for clinicians, caregivers and ourselves to monitor our health and well-being and giving key stakeholders greater access to information about individuals with health challenges, as well as giving consumers valuable tools for prevention and management. Together, these advancements are leading to a convergence of people, information, technology, and connectivity to improve health care and health outcomes." (Other)

3. International exemplars. There were a number of overseas HTA systems offered as exemplars to be considered by the HTA Review: (1) Germany, France, England & Wales (NICE) as examples of systems that achieve rapid reimbursement at reasonable prices; and (2) England & Wales (NICE), Canada (CADTH) and New Zealand (PHARMAC) as systems which have pathways and processes that offer funding for specific technologies, patient (e.g., the Cancer Drug Fund) or population groups.

"The UK's National Health Service process for adding new tests to the national genomic test directory appears to be an efficient, high-throughput process that engages a panel of experts to consider new genomic tests. Elements of this process should be investigated for consideration for adoption in Australia." (Research)

"New Zealand has 2 pathways to address inequity in access: One is the Named Patient Program intended for orphan conditions, such as cystic fibrosis or exceptional condition

criteria - <https://pharmac.govt.nz/medicine-funding-and-supply/make-an-application/nppa-applications/>; The second pathway is a special waiver scheme for drugs that do not meet all the criteria but the physician believes they fulfill the spirit of the special authority - <https://pharmac.govt.nz/medicine-funding-and-supply/make-an-application/special-authority-waiver/>." (Research)

Table 10: Areas of interest for investigation

Stakeholder group	Summary of key inputs extracted
Patient	Digital health (including AI); Access to EMR; Patient registry to boost trial participation; National molecular tumour board.
Industry	Horizon scanning; Interplay between equity and access (poorer access leads to greater inequity); International exemplars in terms of pricing and access (Germany, France and UK); Ethics of denying access.
Research	International HTA processes may not apply; NICE & CADTH elements for review; UK Cancer Drugs Fund; UK Independent Request for Funding; NZ Named patient and special waivers.
Peak	Expand concept of value - examine the clinical, social, and financial value of approving or subsidising a health technology.
Jurisdictions	Co-operative HTA (national); Horizon scanning; Impact of PBS on hospital budgets; Use of IHACPA data in HTA.
Other	AI in HTA; Digital health (exemplars in US, Germany and France)

Abbreviations: AI, artificial intelligence; EMR, electronic medical record; CADTH, Canadian Agency for Drugs and Technologies in Health; HTA, health technology assessment; IHACPA, Independent Hospital and Aged Care Pricing Authority; NICE, National Institute for Health and Care Excellence; NZ, New Zealand; UK, United Kingdom.

4.2.7 Other

Stakeholders varied in their statement of other elements for consideration in the HTA Review (see Table 11), noting some commonality was expressed across stakeholders with respect to the importance of stakeholder engagement. This centred around the importance of consumers and other stakeholders to the HTA process, engaging consumers in a co-design process and ensuring there was feedback on how consumer inputs were being used within HTA decision-making (in part in recognition of the time and resource devoted by consumers to the provision of those inputs).

Table 11: Other elements of note

Stakeholder group	Summary of key inputs extracted
Patient	Assessment of environmental impact; Recognition of stakeholder input (and value).
Industry	Timely access as a means of enhancing societal benefits (productivity gains, reduced carer burden) – these benefits need to be considered.
Research	Specific considerations for rare diseases; Pathway to access new medicines (not funded) via clinical trials; Access to trials is a barrier; Int. sharing might only be relevant for evaluation of clinical evidence.

Peak	Disinvestment processes; Stakeholder engagement process should be co-designed with consumers; Combine HTA for rare disease technologies with jurisdictions (as delivery partners).
Jurisdictions	Lack of understanding of the HTA process; Long lead time for approvals/listing; Lack of transparency and broad stakeholder engagement; Financial criteria review.
Other	Extend periods for market exclusivity/tax credits as incentives for research and development.

Abbreviations: HTA, health technology assessment.

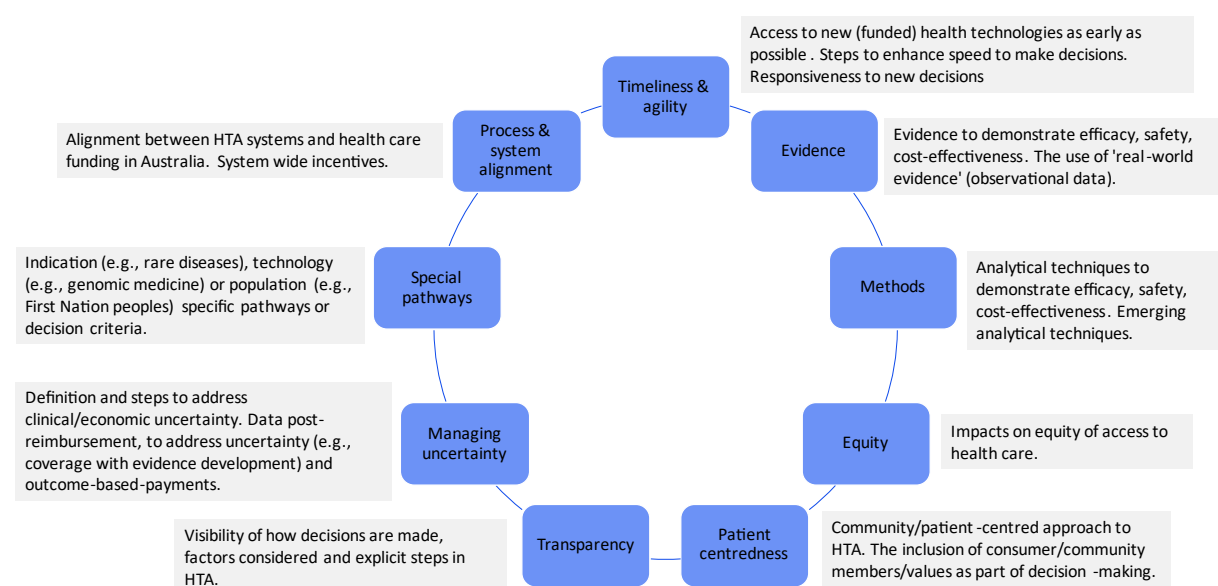
5 Emergent Themes & Options for Change

5.1 Emergent themes

The inductive coding frame applied to the submission inputs is provided in Figure 1. Through a process of inductive review, the deductive coding frame (see Table 2) was modified in the following manner:

- the a-priori theme for International Comparisons was removed as those comparisons generally arose as supporting evidence or exemplars across multiple themes (rather than arising as a specific theme of interest);
- the a-priori theme on Emerging Technologies was replaced by one on Special Pathways on the basis that the latter is more expansive (as it includes technologies, patient groups and indications) but many of the same topics of interest (e.g., the need for 'fit-for-purpose' methods or funding streams); and
- the theme 'Process & System Alignment' was included to capture the importance of the interplay of HTA processes between agencies, various levels of government and various funding sources for health technologies in Australia (e.g., PBS & MBS as a Commonwealth initiative for 'outpatient' medicines, the National Health Reform Agreement, jurisdictional funding and private sector funding).

Figure 1: Resulting inductive codes - emergent themes



Abbreviations: HTA, health technology assessment.

5.1.1 Timeliness and agility

Across the inputs, a key theme was the capacity for timeliness and agility in HTA processes; both in the conduct of assessments and the implementation of decisions made through those assessments. This theme captures the principle expressed by many of the stakeholders for an HTA system that is able to achieve access to new (funded) health technologies for Australian patients as early as possible (following registration).

"In theory, reimbursed access can be achieved within approximately 60 days of TGA registration if: there is parallel processing; a first time PBAC recommendation; and no delays to post-PBAC negotiations. In practice, however, there are very few cases where this is achieved. Updating this analysis to include PBAC recommendations until March 2023 (including listings as of 6 May 2023) results in a similar time to listing of 603 days." (Industry)⁴

"When TGA approval is granted, patients with high priority conditions, such as cancer, are given immediate and affordable access to the drug or test in question....." (Research)

Importantly, the HTA system and the capacity to make decision should remain agile; capable of responding to new technologies and types of evidence quickly to bring rapid access to care. Several

⁴ This quote cites metrics on time to listing from Millar, D, Commercial Eyes Analysis. Presentation by Douglas Millar at ARCS 2022 Conference, ARCS, Australia; 2022 (cited as reference 8 in the Medicines Australia submission).

stakeholders presented the case of access to COVID-19 vaccines and COVID-19 anti-viral agents as exemplars of an agile HTA system, able to respond quickly to a health threat and immediate health care needs.

"This active identification of new treatments for assessment occurs outside of oncology, for example in the quick assessment of antiviral treatments for COVID-19 to respond to the pandemic." (Patient)

5.1.2 Evidence

An important theme arising across the consultation questions and stakeholders was that of evidence within the HTA system. It was recognised that evidence, and the robust assessment of evidence, is critical to demonstrate the efficacy, safety and cost-effectiveness of health care technology. However, the desire for the highest level of evidence was also seen as potentially detracting from the ability of the system to make decisions in a timely manner, or in a manner that did not act as a barrier to indications, technologies or population groups for which evidence from those high standards (e.g., RCTs) was not available. Thus, this theme also reflects the desire for definitions and the use of evidence to become more expansive, to allow for greater use of 'real-world evidence' (observational data) within HTA, along with acceptance for qualitative evidence as part of the evaluation process (particularly in reflecting elements of patient/consumer value).

"Prioritisation of evidence from randomised controlled trials comprises a barrier to timely, equitable access to rare disease health technologies." (Peak)

"Our HTA system is limited in the forms of evidence that it considers when making decisions with a strong focus on large randomised control trials. However, the HTA system should also consider other forms of trial data and include patient-reported outcome and real-world data." (Patient)

5.1.3 Methods

Closely linked to the theme of evidence, is that of the methods applied to the assessment of efficacy, safety and cost-effectiveness of health technologies. There were two critical aspects to this theme; one was associated with modification of the existing methods and analytical techniques as applied to all health care technologies; the other to the recognition that the current methodological approaches may not be fit for purpose with respect to new and emerging health care technologies (such as HST, gene therapies and genomic medicines).

Modifications of existing methods, focused on potential changes to the basis for determining the comparator in HTA, lowering the discounting rate, expanding the range of methods for indirect comparisons of evidence and altering how PICO specifications are derived for HTA of pharmaceuticals.

"The use of 'current standard of care' as a cost comparator for new medicines is a harsh and unrealistic bar to set for diseases in which there is no approved standard of care. ... For cost comparison to be made against off label off patent ineffective unapproved standard of care is simply wrong, and as a result Australian patients are suffering" (Research)

Modifications of methods to accommodate new and emerging technologies highlighted that there are challenges in assessing cost-effectiveness for technologies with high up-front, one-off costs, but a long stream of benefits, those where the benefits may extend beyond health outcomes (such as might occur with genomic medicines and associated diagnostics), and where care may be directed at a molecular level rather than an indication (e.g., pan-tumour therapies).

"The value of knowing should be applied as a principle in all HTA policies and methods."⁵ (Peak)

5.1.4 Equity

The impact of the HTA system on equity of access to health care emerged as a strong and important theme throughout the consultation submissions. Inequity of access manifests in a variety of ways through the HTA system and its operations, including: not accounting for the needs of First Nation peoples; perceived differences in the applicability of HTA processes to different clinical settings (e.g., rare diseases, end of life conditions, prevention); differences in HTA and its implementation across jurisdictions; restricting access to specific cohorts (e.g., those with a specific mutation) within an indication, when there is potential for all patients (absent that mutation) to benefit; not accounting for geographic locations (e.g., metropolitan, rural and regional areas) in reimbursement decision-making; and differences in capacity to access care prior to reimbursement.

"PBS listing is not based upon prioritisation of medication access to those most in need based upon medical criteria and/or non-medical condition (e.g., remoteness). Furthermore, there are limited mechanisms in place to ensure that Aboriginal and Torres Strait Islander people have

⁵ For a definition of the value of knowing and its application in HTA, see:
[http://www.msac.gov.au/internet/msac/publishing.nsf/Content/E0D4E4EDDE91EAC8CA2586E0007AFC75/\\$File/MAC%20Guidelines-complete-16-FINAL\(18May21\).pdf](http://www.msac.gov.au/internet/msac/publishing.nsf/Content/E0D4E4EDDE91EAC8CA2586E0007AFC75/$File/MAC%20Guidelines-complete-16-FINAL(18May21).pdf)

preferential access to life saving or essential medications, despite evidence of the need to access these medications e.g. higher proportion of negative outcomes and/or higher prevalence of disease state as compared to the general Australian population. NACCHO also notes that the long timeframes for MSAC, in particular, has real world implications for equitable access to much needed services by Australians. A further barrier to equitable access is the lack of clarity regarding de-listing of PBS medication process prior to PBAC advice being sought, and lack of clarity regarding options available if the Department and sponsor do not reach agreement, if Department cannot find another supplier or sponsor wants to de-list the medicine. The lack of involvement with service providers, peak bodies and key stakeholders as part of price negotiation for items being listed on the PBS and MBS may create an unnecessary barrier to equitable access." (Patient.)

"Economic inequity is also an issue. Most cancer patients, for example feel the economic impact of treatment, with many now bearing a higher proportion of costs for the medicines they need through the private market and crowdfunding." (Industry)

"...non-medical condition listed (i.e., remoteness) as part of eligibility criteria for COVID-19 antivirals. This serves as one example as to when equity has been considered as an eligibility criterion." (Patient)

A subtheme within equity was that of the ethical implications of decisions made by the HTA system. This theme arose through a number of avenues: that the delays arising in relation to access to therapy disadvantaged current patients relative to future patients, particularly in acute, severe conditions (e.g., advanced cancers); and that there may be mismatches between the use of HTA (statistics) to assess outcomes and benefits as derived from medicines.

"Going back to bioethics we can consider a prominent author, statistician philosopher - Nassim Taleb. He makes a very good case for the mismatch of medicine and statistics, which was developed to substantiate the hard sciences initially. In humans there is too much complexity and diversity - statistics is too inadequate to capture it (with the exception of huge population blockbuster breakthroughs). He makes a very good argument for aspiring to true precision medicine and person-centred medicine and research. Deep investigation and analysis of the whole person and place in a cohort/community and the various actors doing that investigation of both the patient and the environment/ambiance, will be produce quality treatments that sets Australian research apart." (Other)

"It seems as if current HTA processes are primarily concerned with how to best spend taxpayer funds in order to provide cost-effective health care for future generations of patients, rather than the patients of today." (Research)

5.1.5 Patient-centredness

Closely aligned with the theme of a system that achieves equity of access, is one that is patient-centred in its approach to HTA. Core to this theme is the principle that decision-making in HTA incorporates patient/community values (and does not rely solely on clinical and economic endpoints); it is therefore closely aligned to the theme of evidence and expanding the nature of evidence included to be more inclusive of patient focused values as part of HTA. Moreover, a patient-centred approach to HTA was seen as one in which the process of HTA aligned with community/patient values (the notion of community co-design of HTA) and that community/patients had a clear understanding of how decisions were being made (aligning with transparency). Ultimately, a patient-centred system was seen to be one in which we "enable the right medicine to get to the right patient at the right time".

"To develop a truly 'person (or patient) centred' system, the industry and government need to work together to remove any unnecessary barriers that delay access and enable the right medicine to get to the right patient at the right time.....Arguably, this gets to the core of what a person-centred system is meant to be – as treatment decisions are left to the patient and physician without any external barriers or constraints." (Industry)

"Current HTA policy and methods make it difficult to bring a strong and useful consumer perspective into the decision-making process." (Patient)

5.1.6 Transparency

An overarching theme affecting many aspects of the performance of the HTA system was transparency with respect to how reimbursement/pricing decisions are made, the factors (evidence) that are considered in making those decisions, and in some cases understanding the overall steps in HTA. While many stakeholders considered that the process for HTA in Australia was generally well described, it was not always clearly understood.

"...don't have good visibility for how the sausage is made and where the problems are coming from". (Patient)

For some, the language used in describing HTA processes and requirements was overly technical, obfuscating the nature of that process. For others, there was a lack of information provided on specific aspects of the process (such as how evidence was being combined or weighted in reaching decisions about reimbursement/funding) or without public visibility, impeding engagement with the system and potentially resulting in poorer access.

"The DoHAC process for stakeholder consultation, particularly the language used in the Terms of Reference to guide submissions, is too complex and opaque to effectively engage and solicit the views of consumers. Ideally such a process should be co-designed with consumers from the ground up and be part of a longer-term investment in community engagement in national medicines policy." (Peak)

"The HTA process being conducted behind closed doors without meaningful public input can act as a barrier to the earliest possible access." (Research)

5.1.7 Managing uncertainty

Throughout the consultation submissions, it was recognised that a key matter of concern for stakeholders in the HTA process is the consideration of how best to manage uncertainty with respect to clinical outcomes, value for money (cost-effectiveness) and the overall financial impact (i.e., cost to patients, Government and the jurisdictions) associated with health care technologies. Stakeholders noted that while there are approaches to addressing uncertainty (clinical and economic) within the system currently, particularly in the form of managed access programs, they tend to be underutilised. This, coupled with a conservative stance in relation to how uncertainty is viewed, results in delays in reimbursement and pricing decision-making while steps to address clinical/economic uncertainty are agreed to throughout the HTA process.

"One significant barrier to timely access in the Australian HTA system stems from conservative views regarding clinical and economic uncertainty. Decision-making processes often prioritise a cautious approach, particularly when it comes to adopting innovative technologies. In practice this means evaluators will select the most conservative estimate of treatment effect, rather than the most likely." (Research)

Stakeholders discussed a number of avenues, including enhancement of the existing Managed Access Program via the PBAC process (see Options for Change), largely focused on making more use of

observational data for the assessment of clinical or economic uncertainty in the post-reimbursement (e.g., coverage with evidence development) or as the basis for outcome-based-payments.

*"....supports the Managed Access Program (MAP) and would like to see this enhanced. This would allow for patients to access medicines earlier, once certain parameters have been met."
(Patient)*

5.1.8 Special pathways

A theme that emerged strongly throughout the consultation submissions was that HTA processes are not a one size fits all proposition, that it may be beneficial that special pathways are developed for the consideration of technologies that are specific to certain indications, therapeutic areas or population groups. Most notably, current HTA processes were seen to disadvantage access to care for patients with rare diseases largely on the basis that the evidence 'required' in HTA, RCTs, was typically not available for these conditions, and the associated treatments were often of a very high unit price (which was ascribed to the small underlying patient population).

"Another unintended consequence of the rigidity of requirements for evidence and the relationship between evidence and cost is such that rare indications, with small patient populations, which do not have RCT data to support listings are rejected on the basis of single arm studies, or not brought to market for assessment at all because companies know they won't be successful, or if they are, it's not at a price that makes it worthwhile." (Patient)

Similarly, existing processes did not sufficiently prioritise access to care for First Nation peoples and did not establish mechanisms that allowed for technologies to be considered for reimbursement, other than via the standard submission pathway.

Finally, consultation submissions noted that the rapid pace of development in terms of digital health (including AI), in cell and gene therapies, and genomic medicines, necessitated new approaches to HTA for those technologies and therapies in order to capture the full suite of their costs and outcomes. The rapid pace of digital health innovation and the scope of technologies in which it is being applied alters how HTA might conceptualise and value the outcomes of care. Changes in genomic medicine (i.e., targeting the molecule rather than the patient at the intervention level, and the genetic profile rather than a specific mutation at the diagnostic level) suggest changes in how HTA evaluates these therapies for reimbursement purposes. Similarly, advancements in HST call for changes in how those therapies are

evaluated and funded, particularly with respect to the consideration of costs relative to the uncertain clinical benefit, and how the dual clinical and economic uncertainty is managed.

"The TGA registration process determines whether the product is safe and fit for their intended purpose but does not have the same scope to appropriately support public funding arrangements as compared to reimbursement HTA committees. In particular, this should be cautioned for therapies approved via the fast track approval pathway that only have preliminary clinical data to support assessment. It is important to moderate the risks associated with early access before we understand the short- to medium-term benefits. Continued evidence generation under a clinical trial framework or special access scheme is recommended in these cases to enable patient access and the collection of further data to support decision making within the local Australian context." (Jurisdiction)

5.1.9 Process and system alignment

Closely linked to the theme of special pathways, is the consideration that assessment and funding of health technologies in Australia is currently relatively siloed between various Commonwealth and jurisdictional authorities, but that there is need for the Review to address the overall process and system alignment, with respect to consideration of evidence, implementation and funding. This theme draws together a number of key elements. First, currently recommendations are made by Commonwealth HTA authorities that have implications for implementation or funding by the jurisdictions (or even patients, e.g., rescheduling medicines to be over the counter rather than PBS funded). This may result in differential access to care by patients across the country, and has unintended consequences with respect to health care funding arrangements. In addition, stakeholders - particularly the jurisdictions - felt that the Review offered an opportunity to harmonize the approach to HTA in Australia, across agencies and levels of government.

"Due to the lack of integration of HTA processes between the states/Commonwealth, technologies funded through mechanisms other than PBS/MBS (hospital funding/compassionate access/pharma funded/self-funded) may be delivered inconsistently or inequitably, with patients in some jurisdictions and hospitals able to access treatments while others cannot." (Peak)

Second, in seeking to reform HTA and to deliver a system that is more timely and achieves better equity for access, for example, it is important that those metrics (timelines and access) can be reliably measured. However, a number of stakeholders identified that currently it is not possible to routinely

observe those metrics with respect to the performance of the system (particularly with respect to timeliness).

"Currently, it is difficult to determine or even debate which elements of Australia's reimbursement system are working effectively, as the Australian Government does not routinely and systematically collect data on the performance of the PBS." (Industry Organisation)

5.2 Options for change by theme

The options for change as put forward by the stakeholders are presented within this subsection, stratified by theme (noting that any given option for change is likely to affect multiple themes) and grouped according to the main subject area within theme. The options included are those for which some detail of how the change might be implemented has been provided by the proponent; all options as proposed by stakeholders are provided in Appendix 4. Options for change are presented without amendment from what has been proposed by stakeholders (these have not been marked as direct quotes to improve readability), without commentary on the practicality or otherwise of their implementation and should not be interpreted as an endorsement of those options by the authors of this report.

5.2.1 Timeliness & Agility

Options to enhance timeliness and agility within the system focused on introducing new approaches to the process for determining whether technologies are reimbursed and at what price, altering existing processes to deal with evaluation issues as they arise, and recognising the importance of compassionate access programs in facilitating earlier access, notably:

- Introduce 'streamlined listing processes':
 - i. When a therapy is already internationally approved and will only result in Commonwealth expenditure of <\$10M in AU, then these should be subject only to a direct price negotiation.
 - ii. There could be a triaging system which allows a price benchmark – perhaps a percentage of the German price - for new chemical entities or therapies in disease areas with high unmet clinical need (similar to the Innovative and Licensing Access Pathway in the United Kingdom) to allow for immediate access while permanent pricing and post-market requirements are negotiated over a 6–12-month period.
 - iii. When TGA approval is granted, patients with high priority conditions*, such as cancer, are given immediate and affordable access to the drug or test in question. (Note that

TGA approval is often fast-tracked as a result of recent changes to their processes - a paradigm that has not been significantly taken up by the PBAC, but hopefully will be considered as part of the current HTA Review). When such patients are given immediate access to the innovation in question, the PBAC, MSAC, DoH and the sponsor are then given 2-3 years to resolve whether there is sufficient evidence of cost-effectiveness to allow permanent listing on the PBS. If that can't be realised, the drug is effectively delisted and is no longer available to future patients. (*High priority conditions will need to be defined but one such definition might be 'life-threatening illnesses which are inherently unstable over time (e.g., Cancer), that generally worsen progressively and for which treatments that work in one phase of the disease may be much less useful in subsequent phases of an illness'.)

- iv. ... may be solved by requiring international transparency on base pricing and having Australia agree to accept the base price for all registered drugs immediately from registration i.e., able to PBS subsidise at registration. Higher prices can be sought subsequently if new evidence proves them to be cost-effective at a higher price and price reductions will be required if not cost-effective at base price when there is a mandatory HTA at say 12 months post registration, with price changes flowing from 2-year point. Commercial agreement is required at entry will lay out rules for both sponsor and Government on these post-marketing price changes
- v. For new technologies or those with substantial benefit it may be possible to have an interim listing process – where PBAC has recommended the therapy, and the applicant wants to proceed based on the parameters outlined by the PBAC (such as risk sharing arrangements (RSAs), and special pricing arrangements (SPAs) etc) – where listing is highly probable but requires further negotiation. During this process, an interim listing may be possible, where the applicant agrees the interim listing would be for a specified period, and after that period (or if the applicant does not want to continue the PBS listing process), the applicant is responsible, both for supply and cost, for all “supply only” scripts for the prescribed population during the interim listing period. This would guarantee the applicant does not prolong negotiations to sustain the status-quo of the interim period prescribing conditions, while also allowing access to therapies for patients in need. However, the Department would also need to put in place additional safeguards for the applicant – to ensure that any delays in negotiation due to the Commonwealth do not cause the timing of the interim period to expire, creating a significant risk for the Manufacturer, and a disincentive to use such a system. These safeguards could include being transparent with timelines and decisions on the

Commonwealth's side as well as mandatory extensions of deadlines if the Commonwealth does not address the required issues in time.

- vi. Recalibrate the milestone requirements for parallel processing, so that PBAC recommendations are not delayed due to misalignment with the TGA Delegate's Overview. This could be achieved by re-anchoring PBAC consideration to the end of the TGA evaluation phase at Milestone 5 or working with the TGA to bring the timing of the Delegate's Overview forward by several weeks.
- vii. For incremental, marginal products, implement a more limited evaluation process (no need for a full HTA) - products can be listed as cost-minimisation.
- Creating more opportunities for communication/interaction during the evaluation process:
 - i. Develop a 'Rapid Response Team' within the DoHAC HTA team that can work quickly with sponsors between the formal Committee Meetings to quickly resolve and expedite issues to improve submission to listing times.
 - ii. Real-time exchange of information between Sponsor and Evaluator during submission evaluations, so that the most comprehensive assessment is provided for PBAC consideration. Building on the existing plans for an information exchange pilot, this could be achieved by allowing Evaluators to request further information and clarification from Sponsors in real-time via the Health Products Portal (HPP). There should be provisions for Sponsor hearings at ESC meetings. Requests for specific input during evaluations could also be sought from nominated patients and clinicians to reduce the number of resubmissions and deferrals, particularly for new disease areas or where treatment pathways require clarification.
 - iii. Having the opportunity, as is the case in other countries, for face-to-face discussions with the DoHAC (and/or PBAC) to finalize the financial estimates related to PBS listing of the medicine after a positive recommendation is provided by PBAC. This would avoid the need for resubmissions to PBAC with subsequent delays to patient access.
 - iv. Provide option for Sponsors for a "stop clock" during the (evaluation) response period or to preselect different response times (during evaluation to allow for longer response time or to submit additional evidence).
- Compassionate access:
 - i. Improve rules and regulations surrounding compassionate access programs used by sponsor companies during the HTA process in Australia to better protect consumers. Consider a co-funded compassionate access program for patients that need urgent access to a new treatment or technology. This could include a government co-payment

for the treatment which would be refunded by the sponsor company if the treatment is not successfully listed within an agreed time period. To improve equity of access, this program should provide incentives to support patients in regional, rural, and remote Australia to gain access to the new technology or treatment.

- ii. Companies should be reimbursed/or given “credit” towards rebates for compassionate access once reimbursement is obtained as an incentive for broader compassionate access programs or Australia should adopt a reimbursed Early Access Scheme similar to the United Kingdom.

Recognising that timeliness for access to reimbursed medicines is also affected by pricing processes, options for change with respect to price negotiations were made:

- i. Reinstatement of a pricing methods manual as used prior to 2014 to ensure transparency and predictability of negotiated PBS prices.
- ii. Introduce an ability to negotiate price based on bulk discounts/cost savings elsewhere in the health system.
- iii. Consider an independent arbitration mechanism to progress listings for medicines that are recommended by the PBAC where agreement between the sponsor and the PBAC cannot be met.

Finally, in order that timeliness and agility can be monitored, stakeholders suggested options with respect to establishing appropriate system metrics:

- i. Set benchmarking targets for submission to listing times and transparently report back on any reasons for delays. This would make the PBAC and sponsor companies more accountable to the community for any delays in listings.
- ii. .. recommends the adoption of the German and UK models (*for reimbursement*) and places a legislative requirement not exceeding 180 days from approval to prescribing. Once a treatment has received TGA approval, it is considered safe and effective, and could be entered into an early access programme to enable earliest possible access for the people.
- iii. PBAC (or equivalent) should collate and publish annually aggregated information about the ICER ranges accepted for listed medicines by broad therapeutic groups (for example, oncology, non-oncology, rare diseases) to allow tracking of the proportion of medications that have been accepted with ICERs in each ICER range.

5.2.2 Evidence

Options for change addressing perceived issues with evidence were clustered on two key areas: expanding the type and scope of evidence (raised variously across the stakeholder groups), and on the capacity to generate data. Options to expand the type and scope of evidence largely centred on introducing processes that allowed for the collection and assessment of 'real-world' observational and lived-experiences data:

- i. Collect real-world evidence directly from patients through patient-reported outcomes, registries, and patient advisory groups
- ii. Adopt guidance for designing and conducting real world evidence studies for consideration by the PBAC and MSAC, aligned to guidance developed in other countries (notably, the UK and Canada)
- iii. Emphasise the increased use of lived experience and qualitative data, including patient reported outcomes and quality of life data. An example is Project HERCULES in Europe, led by Duchenne UK, which includes the development of patient-reported quality of life measures and considers quantifying the burden of illness.

Options were also put forward with respect to strengthening the capacity within Australia to generate data for HTA:

- a. Increase funding to support domestic clinical trial capacities.
- b. Federal investment into a coordinated national program of linking health data for the purpose of improved oversight over current patterns of care and more accurate modelling.

5.2.3 Methods

Consistent with the inputs from stakeholders, there were a number of options put forward regarding changes to the methods applied in HTA, notably with respect to: ICER considerations and decision-making (including the factors considered); horizon scanning; choice of comparator and specification of the PICO; discount rates; indirect comparisons and the use of reference models.

Many of the options were directed at altering the perceived thresholds applied by HTA agencies, and the factors considered in making decisions about reimbursement:

- i. Apply higher ICER threshold for rare/ultra-rare diseases, those with complex manufacturing (e.g., HST).
- ii. Review requirement for lower ICERs for preventative interventions (the current approach of an ICER of \$15,000 per QALY disadvantages vaccines).

- iii. PBAC's willingness-to-pay threshold should reflect the Australian Governments' own advice on the value of life per the recommendation by the Office of Best Practice Regulation (\$227,000 per statistical life year).
- iv. The Minister for Health and Aged Care provides clear direction to the PBAC that PBS budget impact considerations remain a decision for the Commonwealth Government, and should not inform, nor influence, PBAC recommendations to the Minister.
- v. Broader consideration of value (including societal), esp. for rare diseases - introduce Social Return on Investment analysis.

The absence of coordinated horizon scanning was seen as a gap in the system currently, with a number of options put forward on the types of horizon scanning models that could be adopted:

- i. Create horizon scanning evaluations (similar to CADTH) - which also incorporates whether the medicine is available for that indication in Australia through local clinical trials, the Special Access Scheme or compassionate access. These analyses will then help identify treatment gaps and provide a better understanding of the limits of compassionate access for Australians.
- ii. Establish a horizon scanning activity, as previously undertaken in Australia by HealthPACT, or as undertaken by the NIHR Innovation Observatory in the UK. This activity would provide local stakeholders with valuable information on new and emerging therapies and enable a planned assessment of new technologies based on evidence and local needs. Subsequent to the Solomon review, the Health Technology Opportunity Scan provided to AHMAC by NSW Health in December 2017 (the Menzies review, provided to AHMAC at their meeting on 8 December 2017) provided options for a nationally cohesive approach to HTA. This approach would ensure effective cooperation across all jurisdictions and reduce duplication of work and included a horizon scanning activity as an important method to actively identify new and emerging therapies.

Options for change of the current technical processes and inputs with HTA focused on the following:

- i. Choice of comparator (PBAC):
 - a. The PBAC Guideline definition of main comparator should be incorporated into the National Health Act definition of 'alternative therapies'.
 - b. The comparator should be the therapy(ies) most likely to be replaced in clinical practice by the new intervention, aligned with other HTA bodies and good HTA practice. This is consistent with the earlier interpretation of the National Health Act (pre-2015). Where there are multiple comparators, the economic assessment should calculate a weighted

average price for the new therapy based on the proportion of use that it replaces for each of the comparator therapies.

- ii. Discount rate: The base case discount rate should be reduced to be in line with comparable markets (e.g., 1.5% at NICE) and the time horizon used in the economic evaluation should reflect the nature of the disease and intervention under consideration.
- iii. Indirect comparisons: For the purpose of demonstrating clinical superiority and cost effectiveness when head-to-head trials are not available, adopt the methodology accepted in other HTA markets (e.g., NICE, CADTH).
- iv. PICO ratification/agreement:
 - a. PICO Ratification: Alignment on HTA parameters prior to PBAC submission lodgement, to help reduce the number of resubmissions, and to resolve funding pathways and implementation plans for complex technologies and areas of high unmet need as early as possible. This could be achieved by replacing the current optional pre-submission meeting process led by the DoHAC which has limitations around stakeholder attendance, restricted time for consultation and is not a formal part of the HTA process. Instead, a more comprehensive optional pre-submission protocol ratification process could be implemented for complex technologies or areas of high unmet need led by a subset of Committee members, for example the relevant Discussant, PBAC Chair and ESC. It could include elements such as PICO, financial and economic model parameters as well as the opportunity for patient, carer, and clinician input.
 - b. Adopt an alternative pathway for determining PICO as part of PBAC submissions (akin to the current MSAC application process; it effectively determines the appropriate PICO for the medical service/technology) or at least a binding decision between the Department and the applicant should be established prior to commencement of the assessment to determine the appropriate population and comparator, and mitigate conflict between the assessment and the Committees at the decision-making stage.
- v. Reference models: Use disease reference models for economic analyses that are informed by registry and health administrative data (i.e., RWE) to account for the impact of novel therapies beyond a single stage of treatment.

5.2.4 Equity

Options to address equity of access were raised with respect to access to care for First Nation peoples, changing the nature of the system to make it easier for non-industry stakeholders to lodge submissions, recognising that cost to patients is a barrier to equity of access:

- Access to care for First Nation peoples:

- i. Automatic exemption of PBS Aboriginal and Torres Strait Islander list for statutory price reductions.
 - ii. Automatic fee waiver for PBS listing requests which may have a substantive positive impact on access for Aboriginal and Torres Strait Islander people, inclusive of PBS listing for Aboriginal and Torres Strait Islander people.
 - iii. Negotiation/engagement (face-to-face) with NACCHO prior to HTA implementation.
 - iv. .. inclusion of NACCHO in pipeline development and horizon scanning activities, where future technologies that may benefit Aboriginal and Torres Strait Islander people may be supported by stakeholders and government to improve the chance of accessibility.
 - v. A managed access pathway for preliminary PBS listing where a medication without standard clinical trial effectiveness data could be approved for preliminary listing for Aboriginal and Torres Strait Islander people under PBS, with ongoing PBS listing based upon real world evidence. We are concerned that managed access programs (MAPs) alone are not a sustainable means to supporting equitable access of medications for Aboriginal and Torres Strait Islander people. The proposed preliminary PBS listing pathway approach builds upon the approach taken by PBAC in relation to listing ipilimumab on the PBS with the condition that the sponsor work with clinicians to provide two-year overall survival data of patients who accessed ipilimumab over 12 months. It is suggested that the preliminary PBS listing pathway consider conditions such as providing patient specific data (such as overall survival data, adverse event data, hospitalisation data), sentinel site data, supply data - where applicable. Specific example: PBS listing of Ozempic, Trulicity and other medications for diabetes for Aboriginal and Torres Strait Islander people under 18s (noting that current use is off label). This pathway could also be informed by recent international guidance available around RWE developed by ISPOR, EMA, UK NICE, Health Canada and International Council of Harmonisation. Importantly, this preliminary PBS listing pathway could support existing health inequities whilst also acknowledging and valuing Aboriginal and Torres Strait Islander cultural practices and perspectives e.g., through consideration of Aboriginal traditional medicines.
 - vi. Incorporating forcing functions to ensure that Aboriginal and Torres Strait Islander people have preferential access to life saving (or essential ongoing) medications such as prioritising access for Aboriginal and Torres Strait Islander people with reduced PBS restrictions as compared to the general Australian population, similar to the approach taken by New Zealand's Pharmac.
- Non-industry submissions:

- i. Establish a fund to support (non-industry) health professionals, peak bodies and consumer groups to sponsor registration and reimbursement applications for certain health technologies.
- ii. Modify systems to make it more feasible for individuals/ stakeholder organisations (e.g., NACCHO) to apply to PBAC/MSAC.
- iii. As an alternative to a sponsor lead process, a commissioning approach could be applied for medicines with an identified need. For example, nicotine gum and lozenge were de-listed in April 2023 when the only PBS sponsor was unwilling to accept the PBS price, perhaps an open tender process could be run to find a replacement at an acceptable price (similar to PHARMAC).
- iv. Establish a relevant and specific communication channel and require when necessary fee-free state initiated submissions when PBAC listing restrictions do not reflect current clinical guidelines (e.g., dose optimisation of infliximab in inflammatory bowel diseases), or when a medicine is recommended in international guidelines but unlikely to be TGA-approved or PBS-funded (e.g., delamanid in multi-resistant tuberculosis) or when an application was rejected by PBAC and the company did not reapply.
- Reducing cost barriers to patients: Requirement that if PBS listed medication is not available, but the same sponsor has a medication available with a brand price premium, that brand price premium cost be withheld.
- One-Stop-Portal: Establish an online portal that provides information for consumers on all available technologies; what is available for whom, how and where can it be accessed, and what is the cost to the patient associated with accessing care.

One patient organisation (Asthma Australia) also offered a number of options specific to the improving equity of access to asthma therapies:

- Increase equity in access to asthma preventer medicines by:
 - a. Establishing a communications and education support service for health care professionals and consumers in relation to the quality use of medicines to ensure they are used 'safely, optimally and judiciously, with a focus on informed choice'.
 - b. Developing mechanisms to enable the PBS to provide higher co-payments for more costly medicines – such as inhaled corticosteroids - that prevent chronic conditions from deteriorating and that keep people out of hospital and emergency departments.
 - c. Making costs of medications visible to prescribers by providing real-time data on the relative costs of therapeutically equivalent medicines and by cost per dose.

- That the Australian Government include additional item numbers on the Medicare Benefits Schedule for pulmonary rehabilitation for people with complex chronic respiratory illnesses.
- That DoHAC identify overuse of oral corticosteroids in asthma management as a priority, and reduce it by working with:
 - a. Sponsors to create an oral corticosteroid rescue pack with 10 x 25 mg tablets only (with the current provision of 30 tablets to be indicated only for use by people with severe asthma who require OCS for asthma control)
 - b. PBAC to reschedule oral corticosteroid use to authority prescription only, and
 - c. PBAC to review processes determining access to monoclonal antibody therapies to ensure that people with severe asthma are not required to use potentially toxic cumulative doses of oral corticosteroids when biologics would be more a beneficial treatment for them.
- That the Australian Government enable access to spacers and masks under the PBS to support equitable access given their importance in effectively and efficiently administering asthma medicines, particularly to children.

5.2.5 Patient-centredness

Options for change with regard to achieving patient-centredness focused on improving the use of consumer and patient inputs, enhanced consumer representation and engagement with HTA, including building capacity among consumers/ the community to contribute to and participate in HTA:

- Consumer and patient input:
 - i. Seek targeted input from people with lived experience and their caregivers rather than relying on people to submit comments via a portal.
 - ii. Provide independently reviewed plain language, transparent summary documents for each listing which are aimed at consumers and consumer organisations. These summary documents should provide factual information and links to key data sources that are going to be relied upon during the HTA Assessment process.
 - iii. Provide meaningful feedback on the consumer comments process and how this has or has not supported the Committees in their decision-making processes. This needs to go beyond a short mention in the PSD and must make mention of how the consumer perspective and lived experience was considered in the decision-making process. Committees need to be honest as to whether consumer feedback has been valuable and explain why.
 - iv. Develop a register of accredited consumer groups that have been trained and commit to ongoing development to contributing to HTA processes. HTA should consider providing

- grants to accredited consumer groups to help gather independent consumer input from their community, especially for high impact listings that require significant time and resources.
- v. Provide training and examples of what good consumer comments look like and provide opportunities to educate and empower consumer groups on how to add value to HTA processes. Co-design with consumer/consumer groups a suite of best practice guidelines on how to add value to the HTA processes through the consumer comments process.
 - vi. That an Enhanced Consumer Engagement Process be co-designed with patients, as agreed to in the Strategic Agreement, which includes an opportunity to participate at PBAC meetings as part of discussions about clinical evidence, to elevate the patient voice in decision making.
 - o That HTA bodies provide patients with summaries about how consumer inputs are considered as part of decision-making processes;
 - o HTA bodies better incorporate patient and carer input by having reference to a broader subset of utility values such as patient preference, patient reported outcome measures, and discrete choice methodology.
 - vii. Conduct Patient/Caregiver Surveys and Interviews: Conduct systematic surveys or interviews to gather patient and caregiver perspectives on health technologies during their evaluation. These can include questions about treatment outcomes, quality of life, and other relevant aspects. The UK's National Institute for Health and Care Excellence (NICE) engages patients and caregivers through public consultations and surveys to ensure their perspectives are considered during HTAs.
- Consumer representation and engagement:
 - i. The number of patient / consumer representatives on HTA committees needs to be increased to at least 3 representatives per committee.
 - ii. Establish Patient and Carer Advisory Committees: Create formal committees consisting of patients, caregivers, and patient advocacy groups to provide input and insights during HTAs. These committees can review and comment on HTA submissions, participate in decision-making processes, and provide feedback on the patient experience. Sweden has successfully implemented patient involvement in HTAs through patient advisory committees, such as the Swedish Agency for Health Technology Assessment and Assessment of Social Services (SBU) and the Dental and Pharmaceutical Benefits Agency (TLV).

- iii. Sponsors should be empowered to increase patient involvement across the breadth and depth of the HTA process. This could include working more closely with patient organisations to alert them of the sponsors intention to:
 - o seek registration of a new medicine with the TGA at the beginning of the process. The information provided should include summary information about the eligible patient population, plain language detail as to the clinical studies, key clinical outcomes and what they mean for patients and any safety considerations.
 - o submit to PBAC. Including the provision of any additional information not provided at the time of registration with the TGA. We note that this is currently standard practice but is often reliant on relationships rather than being process driven.
 - o withdraw or somehow change existing listings on the PBS, so that patients can be alerted in a timely way.
 - o include a section in their submission to PBAC that describes interactions with the patient population such as focus groups, discussions, surveys, etc about the impact of the new medicine on quality of life, (including the impact on mental health, carers, employment, etc) and share this information with the patient organisation (as it may have a bearing on any submission they will make to PBAC).
 - iv. Ensure consumer hearings and stakeholder meetings include the presence of at least two people from the patient community impacted, in addition to any representatives from the patient organisation.
- Enabling consumer input (capacity building):
 - i. Compensate or reimburse any involvement of volunteer consumers in HTA processes including sitting fees, travel and accommodation costs and ensure protocols for consumer involvement are implemented; this involvement should be appropriately supported so that people with cognitive impairment are able to meaningfully participate.
 - ii. Create pools of funding for those patient organisations with limited resources to enable their development of submissions and their own consumer engagement processes.
 - iii. Terms such as ‘medical intervention’, ‘indication’, ‘comparator’ and ‘public health significance’ are not terms that the majority of the general population know. This form should be simplified, keeping health literacy in mind. For us to be able to collect more valuable information, it would be good if the consultation period was not during the holidays or during the busiest time of year (Christmas). Provide funding for patient groups or consultation with consumers. Weight the feedback of patients/consumer groups with

- more importance than other aspects of the process. Is there an option for consumers/patient groups to provide their feedback via telephone with a consultant?
- iv. The capacity of the Consumer Evidence and Engagement Unit (DoHAC) should be increased; the unit's remit should be expanded to support HTA applications by non-commercial organisations where there is no commercial incentive for a submission.
 - v. Provide education and feedback to assist consumer organisations to understand what constitutes evidence in a HTA setting and how they can best collect this evidence, including feedback on improving the quality and effectiveness of the comments they provide.

5.2.6 Transparency

Options for change with respect to system transparency were aimed at addressing visibility of the decision making process, improving communication about decision outcomes, and exploring the potential for industry sponsors to collaborate prior to lodging applications:

- Decision-making processes
 - i. Sponsors to participate as an observer during the PBAC meeting when their agenda item is discussed. The deliberation of final PBAC recommendations could remain confidential, as would competitor information. Sponsors should also be provided with a copy of Discussant presentations, as well as any communication between the DoHAC, Committee and Evaluators regarding their submission (that is not competitor in confidence). The agenda of all PBAC Executive meetings should be published.
 - ii. All documentation (all submission correspondence; agendas of PBAC Exec meetings) should be published.
 - iii. More clarity and transparency about the approval process is required, so that the stakeholders, including clinicians, the affected community and industry are clearer about the process and the progress of specific proposals. - All steps in the evaluation process should be identified and timelines published. - This includes the timeframes for the public release of recommendations to avoid delays in informing the clinicians and community.
- Decision-making outcomes
 - i. As the general community are reading the PBAC outcomes, the technical information could be written in clearer more accessible language. Separating cost effectiveness from price negotiations would allow the information (recommendations) provided to the community to be far easier to understand (e.g., less about patient numbers and similar details, effectively meaning the community would be less confused and anxious about the process).

- ii. The PBAC minutes should explain the Committee's rationale for each product's accepted ICER (for example, why \$50K/QALY rather than \$70K/QALY and why they thought the ICER was acceptable in each instance). This information should be redacted in the Public Summary Documents. Provide greater structure and transparency in how contextual factors such as severity, rarity and equity are incorporated into funding recommendations (including in how the case specific maximum ICER was determined).
 - iii. All MSAC and PBAC submissions and assessment reports should be published and not restricted by commercial-in-confidence. The evidence-base and assumptions related to decision-making should be transparent to all stakeholders and will assist in local implementation.
- Improving collaboration through transparency: Seek guidance from the ACCC on competition law to enable discussion between multiple Sponsors at time of submission and PBS listing.

5.2.7 Managing uncertainty

Options for change in terms of managing uncertainty concentrated on how managed access programs, including requisite data collection for those programs, could be introduced/reformed:

- i. Prior to recommending a MAP as part of a PBAC recommendation, a feasibility assessment of data collection and analyses required to address outstanding HTA questions should be conducted with the details of the MAP finalized and agreed between the parties. The agreement should include sufficient detail on data sources and data analysis methods, particularly for the use of real-world evidence and clear boundaries for meeting/not meeting MAP criteria. Evaluation of the available evidence should be conducted in a manner specific to the MAP research question at hand applying the 'most-reasonable' interpretation of data collected within a real-world setting, rather than defaulting to the most-conservative or applying strict HTA methodology.
- ii. Establish a pathway of conditional drug reimbursement, whereby ongoing reimbursement after a period was dependent on generation of supportive data from internationally or from Australian use. This could be best achieved via drug access linked to data acquisition via a clinical trial or national registry, with registry funding from the drug sponsor. Consideration should be made to allowing approved and registry-experienced research groups manage such registries given the established research links with the clinicians using these medicines and their data entry staff responsible for currency of data, and familiarity with collecting data pertinent to disease specific endpoints. Such registries could also capture postcode,

- gender, and ethnicity of patients in these conditionally reimbursed programs to track timeliness of equity of access.
- iii. Early access to and funding of novel therapies should be supported by adequate data collection. For example, in the UK the NHS has a range of registries and databases for outcomes collection:
 - o For leadless pacemakers “Clinicians should enter details about all patients having leadless cardiac pacemaker implantation for brady-arrhythmias onto the National Institute for Cardiovascular Outcomes Research database and review local clinical outcomes” (<https://www.nice.org.uk/guidance/ipg626/chapter/1-Recommendations>).
 - o The National Cancer Registration and Analysis Service (NCRAS) in the UK (<https://www.cancerdata.nhs.uk/>)
 - o NHS Digital clinical audits and registries (<https://digital.nhs.uk/data-and-information/clinical-audits-and-registries>).

This usage and clinical data should be shared with relevant stakeholders (e.g., state and territories, clinicians), rather than being collected under commercial-in-confidence agreements. These arrangements should be part of the initial approval and funding agreement.

5.2.8 Special pathways

Throughout the consultation submissions there were a number of options which addressed the potential for change with respect to processes or pathways for specific indications or technologies.

Of these, there were a number of options for change noted with respect to rare diseases:

- i. Reduce cost-recovery charges for rare disease therapies.
- ii. Reduce submission fees for PBAC submissions for orphan-designated drugs.
- iii. Strengthen focus on rare disease via:
 - o stronger orphan drug incentives to bring Australian orphan drug designation in line with other countries, in particular offering advice and assistance to small companies that have not previously interacted with the Australian regulatory system.
 - o a program like the FDA’s ‘Rare Paediatric Disease Priority Review Voucher Program’³ should also be considered. A unified patient registry or network of patient registries is urgently needed for the rarer neurological or neuromuscular diseases and disorders. Could be expanded to include dementia etc. Data capture from existing medical records should be integrated into a resource that allows accurate identification and characterisation of the patient population for clinical trial planning. Could be enabled to allow for expanded access to clinical trials.

- this includes a coordinated independent national molecular tumour board to discuss complex results from sequencing to guide optimal therapy, to bridge the gap between health technology development and successful implementation.
- iv. In accordance with the recommendations of the New Frontier Report, the Australian Government establish a Centre for Precision Medicine and Rare Diseases within the Department of Health and Aged Care (DOHAC). This would:
 - Identify and track HTA applications for orphan drugs through reimbursement pathways so that rare disease specific issues can be identified and addressed.
 - Coordinate HTA applications for rare disease therapies through a single-entry point within the DoHAC.
 - Recognise in HTA guidelines that, for rare diseases, observational data is the best evidence available for decision making.

Similarly, there were a number of options for change offered with respect to the assessment and funding of cell and gene therapies:

- i. In agreement with The New Frontier Report, the Australian Government establish a Centre for Precision Medicine and Rare Diseases within the Department of Health and Aged Care (DOHAC), to ensure that the capacity of the DOHAC is enhanced to provide Australians with timely access to new medicines and novel medical technologies. The Centre should:
 - Provide advice to governments on the establishment of a dedicated HTA pathway for cell and gene technologies,
 - Outline a simplified HTA process for cell and gene therapies, and Review HTA methodology applied to cell and gene therapies, considering international best practice.
- ii. Establish a single HTA assessment body for cell and gene therapies to remove current inconsistencies and complexities to streamline the pathway for patient access.
- iii. Establish a single federal funding source for the product costs of cell and gene therapies, similar to PBS funding of medicines.
- iv. Streamline and, where appropriate, standardise the clinical delivery of cell and gene therapies to ensure equitable patient access and improved quality of life for patients and autonomy for clinicians to best meet the needs of patients under their care.
- v. For funding of HST:
 - Split and cap payments, to mitigate risk due to the immature evidence and uncertainties in therapeutic outcomes, costs and patient numbers.
 - Follow-up review of HSTs approved under the NHRA.

- Consideration for HSTs would be to enable delivery in private hospitals to support broader access and develop mechanisms to recognise and account for patients using private health insurance in block funding arrangements for public hospitals. Over half the Australian population has private health insurance, and patients should have a right to use this as part of their treatment (e.g., choosing their specialist). Permitting the use of private health insurance, while ensuring the appropriate mechanisms are in place to recognise and account for these instances in the average costings, will likely result in improved consumer confidence and potential cost savings for these high-cost therapies.

Options for change were also noted with respect to the processes applied to other specific technologies or pathways:

- i. Life-saving drugs: allow for direct application to the Life Saving Drugs Program (no double process with the PBAC).
- ii. Tumour agnostic therapies: That there is greater consideration of excluding testing costs in HTA, notably when the testing technology is anticipated to be embedded into the health infrastructure in the near term, or when access to testing is covered through other means (ie. patient self-pay or clinical trial).
- iii. Consideration and funding of vaccines:
 - Evaluators for ATAGI should be chosen based on expertise relevant to the vaccine and disease area. Sponsors should be permitted to engage with ATAGI. Allow review of price for vaccines on NIP (without a full submission).
 - If the (separate) ATAGI assessment process is retained, then external evaluators should be selected on the basis of their personal expertise relevant to the specific product and disease area.
 - ATAGI must allow sponsors the opportunity to formally engage with them during the assessment process (adopt similar protocols to the PBAC assessment process).
 - Remove double cost recovery measures that apply to vaccines which are reviewed by both ATAGI and PBAC before they can be listed on the NIP.

5.2.9 Process & system alignment

Options for change with respect to the overall HTA process and system alignment addressed the perceived need to improve alignment of existing HTA committees and approaches in Australia (within and beyond the Commonwealth agencies), align funding for technologies across the Commonwealth and

jurisdictions, building capacity within the system to conduct HTA and reforming processes (beyond those noted above under the other themes) in the conduct of HTA.

Achieving system alignment included the following options:

- i. Consult with the Health Technology and Genomic Collaboration to align with planned implementation of the NHRA 2020-25 long-term reform to develop a nationally cohesive approach to Health Technology Assessment.
- ii. State/Commonwealth HTA processes and the National Hospital Funding Agreement should be reconciled to ensure equitable access to non-PBS/MBS items (e.g., self-funded, hospital funded, compassionate use, etc.), irrespective of health service jurisdiction.
- iii. Share Commonwealth-funded HTA assessments with state/territory jurisdictions; health system-based Drug and Therapeutics Committees (DTCs) do not have sufficient capacity to undertake cost-effective analyses independently. Sharing of assessments by TGA/PBAC/MSAC would foster streamlined and more consistent decision-making, increase equity of access to medicines nationally, reduce opportunity costs and decrease duplication of effort undertaken by individual states, healthcare services and hospitals.

Specific options for alignment of processes related to the consideration of blood and blood product technologies, and medical devices were also raised:

- i. HTA alignment: Medical devices and prostheses
 - o Reference group pricing. The PBS uses reference pricing for generic clusters and for groups of drugs with similar safety and health outcomes that can be used interchangeably (therapeutic groups). Medical devices are assessed on comparators based on the functions of the device, absent the rules imposed by the Pharmaceutical Benefits Advisory Committee (PBAC) on selecting the most suitable comparator. The Government has flagged an intention to regroup the Prescribed List of Medical Devices and Human Tissue Products (the PL) in line with clinical groupings, but this work has been delayed.
 - o Ensuring benefits of competition. The PBS uses two formularies. Formulary One consists of drugs which have only one brand each; Formulary Two consists of drugs which have two or more brands each. When a competitor comes onto the market, prices are reassessed to ensure the consumer benefits from competition. No such mechanism is used for medical devices.
 - o Considerations on pricing. The PBS has a number of rigorous processes to assess economic value, international pricing comparisons, and post-market reviews. Many of

these processes are absent with medical devices, in particular, consideration of international price benchmarks. When setting prices, PBAC has options including reference pricing, cost-plus pricing and other mechanisms to improve public value. Further, the PBS may use risk-sharing arrangements to protect public value, a mechanism unavailable for devices.

- Limitations on usage. PBAC considers the scope for use of the drug beyond any restriction for subsidy, and the extent to which a restriction can be constructed that satisfactorily distinguishes use that is acceptably cost-effective from use that is not cost-effective. In contrast, many items on the PL have been assessed and approved for one purpose but are commonly used for a different purpose. Once an item is on the PL, it must be subsidised by health funds without regard to quality, efficacy, efficiency or safety.
- Regroup the Prescribed List of Medical Devices and Human Tissue Products (the PL) in line with clinical groupings.

ii. HTA alignment: Blood products

- The Department of Health should adopt a protocol for evaluation of blood and blood-related products in-line with that for other pharmaceuticals.
- The Department of Health should provide specific guidance and documentation to explicitly allow a parallel registration-reimbursement assessment process for blood products, and comparable with other medicines.
- The National Blood Authority develop fit-for-purpose processes, to ensure expedited access to new blood and blood-related products. This should include: Development and publication of an appraisal calendar with meeting dates, deadlines, opportunities for input (from sponsor, healthcare professionals and consumers) and notification of outcomes; creation of an independent blood product expert appraisal committee with membership appointments based on expertise in the blood sector, and related therapeutic areas; establish comparable implementation timeframes and transparency in outcomes to PBAC; leverage existing HTA procedural architecture to harmonise processes between the NBA, JBC, MSAC and PBAC to allow a single point of contact during HTA evaluation and to support expedited resolution of issues; and development and publication of clear KPI's, that are benchmarked to international standards.

Options were also raised with respect to the importance of harmonising how health technologies, notably medicines, are funded in Australia:

- i. Provide PBS funding for hospital in-patient prescribing
- ii. Develop a single-funder model for health technologies provided in hospitals to [reduce duplication in the assessment of safety and cost-effectiveness and] facilitate early and equitable access to high-cost and complex medications
- iii. Enable public hospital pharmacies to supply PBS-subsidised medicines for public hospital inpatients to achieve equity and enhance quality use of medicines and medicines safety.
- iv. Enable hospital pharmacists to supply medicines to Indigenous Australians under Closing the Gap PBS Co-Payment Measure.

In recognition of the importance of expert input for the conduct of HTA, the following option for change was noted: Endorsement of the Zimmerman Commonwealth report (2021), 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.”

6 Summary of findings

This report presents a qualitative synthesis of the stakeholder (n=113) submissions to Consultation 1 of the HTA Review. Input from those submissions identified several key strengths within the existing HTA process in Australia, largely with respect to the degree of rigour applied (both in terms of the type of evidence considered and how that evidence is assessed), the inclusion of consumer representatives and consumer inputs into the process, and a perceived flexibility in decision-making which has in many cases allowed technologies to be made available to Australian patients.

However, the input from the submissions noted that that same rigour and desire for a robust process could be impeding timely access to care, with a perceived overemphasis of the importance of data from RCTs and achieving an acceptable ICER was seen as a key factor contributing to delays in timely access to medicines. This was particularly the case with respect to medicines/technologies for rare diseases, or new and emerging technologies for which the current HTA system was not seen as fit for purposed. Moreover, the absence of a harmonised approach to HTA and the implementation of recommendations made by agencies was seen as further hampering timely access to medicines.

The focus on RCTs and the ICER were also seen as contributing to a perception that consumers and patient values are absent from the system. In addition, submissions noted the extent to which the current system faces barriers with respect to the transparency of what is considered in reimbursement decision-making, and the process applied in reaching reimbursement decisions. Stakeholders also

identified a need for a better use of managed access programs, incorporating real-world evidence, as a means of enabling early access to medicines in situations where there is uncertainty with respect to clinical outcomes, economic performance, or both.

This input gave rise to nine key themes: timeliness and agility; evidence (used to inform HTA); methods (applied in HTA); equity of access; patient centredness; transparency; managing uncertainty; special pathways (indications, technologies or populations); and process and system alignment. Stakeholders proffered a large number of options for change across all themes. Those options varied with respect to the degree of detail provided regarding the steps/pathways by which options might be implemented.

That aside, options focused on implementing alternative approaches for subsidising technologies (including subsidising some/all technologies at the time of registration, with HTA assessment to follow); expanding the scope of evidence considered to give more weight to the inclusion of observational data (real-world evidence) both in making decisions about reimbursement and in managing uncertainty; incorporating broader aspects of value, in particular PREMS and PROMS in assessments of technologies; facilitating greater community participation in shaping the HTA system and the process of HTA; moving away from a one-size-fits all approach to HTA to recognise specific technologies and indications; and refining the overarching HTA processes so they are better aligned across agencies, and allow for non-sponsor initiated submission.

Appendix 1: Consultation 1 Survey Questions (Complete Detail)

Page 1 - Elements and features that are working effectively

Understanding the elements and features of HTA policy and methods that are working effectively, will help to ensure they are preserved and continue to provide positive outcomes for Australians.

Are there any elements and features of HTA policy and methods in Australia that are working effectively?

☐ Yes - there are elements or features that are working effectively and should not change.

Are you able to provide detail of any elements and features of HTA policy and methods that are working effectively? Please use specific details where possible.

Are you able to provide details of positive outcomes resulting from Australia's HTA policies and methods? Please use specific examples where possible.

Page 2 - Current or future barriers to earliest possible access

Reducing time to access for Australians so that they can access new health technologies as early as possible is recognised as a shared goal of both the Government and Medicines Australia as agreed under clause 5.1 of the Strategic Agreement.

What are the elements and features of HTA policy and methods that are acting as a current barrier to earliest possible access?

Where possible, please detail:

- Specific examples or experiences
- The specific policy, method and/or mechanism that is causing the barrier
- The group/s being impacted
- The magnitude of the impact
- The group/s in the HTA approval pathway contributing to these issues.

What are the elements and features of HTA policy and methods that may act as a future barrier to earliest possible access?

Where possible, please detail:

- Specific examples or experiences
- The specific policy, method and/or mechanism that will cause the barrier
- The group/s impacted
- The magnitude of the impact
- The group/s in the HTA approval pathway contributing to these issues.

Would you like to provide feasible options or suggestions you have to improve elements of HTA policy and methods that are acting as a current or future barrier to earliest possible access?

Where possible, please detail:

- Specific examples or experiences
- The specific policy, method and/or mechanism being suggested
- The group/s in the HTA approval pathway that will need to contribute to the solution
- The outcome the suggestion is expected to achieve
- Any foreseeable risks or negative impacts the suggested change may have and possible ways to mitigate them.

Page 3 - Current or future barriers to equitable access

What are the elements and features of HTA policy and methods that are acting as a current or future barrier to equitable access?

Where possible, please detail:

- Specific examples or experiences
- The specific policy, method and/or mechanism that is causing the barrier
- The group/s being impacted
- The magnitude of the impact
- The group/s in the HTA approval pathway contributing to these issues.

Are you able to provide details of feasible options / suggestions to improve elements of HTA policy and methods that are acting as a current or future barrier to equitable access?

Where possible, please detail:

- Specific examples or experiences
- The specific policy, method and/or mechanism being suggested
- The group/s in the HTA approval pathway that will need to contribute to the solution
- The outcome the suggestion is expected to achieve
- Any foreseeable risks or negative impacts the suggested change may have and possible ways to mitigate them.

Page 4 - Elements and features that detract from person centredness

Are you able to provide details of any elements and features of HTA policy and methods that may be detracting from person- centeredness?

Where possible, please detail:

- Specific examples or experiences
- The specific policy, method and/or mechanism that is detracting from person-centeredness
- The group/s being impacted
- Details of the impact this is having
- The group/s in the HTA approval pathway contributing to these issues.

Are you able to provide details of feasible options / suggestions to improve elements of HTA policy and methods that are detracting from person-centeredness? Yes

Where possible, please detail:

- Specific examples or experiences
- The specific policy, method and/or mechanism being suggested
- The group/s in the HTA approval pathway that will need to contribute to the solution
- The outcome the suggestion is expected to achieve
- Any foreseeable risks or negative impacts the suggested change may have and possible ways to mitigate them.

Page 5 - Perverse incentives

HTA helps to ensure equitable and sustainable access to safe, cost- effective, and affordable health technologies for all Australians. A perverse incentive is where an element or feature HTA policy and methods may be creating an unintended incentive that results in negative consequences.

Are you able to provide details of elements of features of HTA policy and methods that are causing or could cause unintended consequence or perverse incentives?

Where possible, please detail:

- Specific examples or experiences
- The specific policy, method and/or mechanism creating the perverse incentive
- Details of the unintended outcome occurring or that could occur
- The group/s contributing to these issues.

Are you able to provide details of feasible options / suggestions to improve elements of HTA policy and methods that are creating unintended outcomes or perverse incentives either currently or in the future?

Where possible, please detail:

- Specific examples or experiences
- The specific policy, method and/or mechanism being suggested
- The outcome the suggestion is expected to achieve
- The group/s that will need to contribute to the solution.
- Any foreseeable risks or negative impacts the suggested change may have and possible ways to mitigate them?

Page 6 - Areas for further investigation or analysis

Under section 5.3 of the Strategic Agreement it was agreed that an expert in HTA would be engaged, to undertake an analysis of current methods used by the PBAC, contemporary research and relevant methodologies and purchasing practices used by comparable international jurisdictions as guided by the terms of reference.

Draft versions of initial findings from this analysis will be available to stakeholders through Consultation 2 later in the year.

Noting the overall scope of the analysis from the HTA expert will be in line with the ToR and agreed by the Reference Committee, are there any HTA or reimbursement models, or elements thereof, utilised in other countries that you believe should be considered for potential adoption in Australia, or that it would be good for the Reference Committee to understand?

Where possible, please provide:

1. - Country / Jurisdiction
2. - Details of:
 - a. Which elements of the HTA policy, method, mechanism for suggested for consideration
 - b. Any outcomes that the suggestion is achieving that should be considered
 - c. Any unintended consequences that the suggestion is having or may have if adapted in Australia

Country / Jurisdiction:

Details of: Which elements of the HTA policy, method, mechanism for suggested for consideration; Any outcomes that the suggestion is achieving that should be considered; Any unintended consequences that the suggestion is having or may have if adapted in Australia

Page 7-Other details of importance to the HTA Policy and Methods Review not covered above + document / attachment upload point.

The HTA Review [terms of reference](#) sets out:

- the background to the HTA Review
- how the HTA Review will take account of recent and concurrent reform processes that impact HTA
- HTA Review objectives
- areas under consideration by the HTA Review.

Noting the objectives of the review set out in the Terms of Reference, is there any other information relevant to the Review not provided above that you would like to add?

Would you like to upload any attachments/supporting evidence to your submission?

(File upload point)

Appendix 2: Contributors by Self-Nominated Stakeholder Group

Stakeholder group	List of stakeholders
Patient or consumer organisation	<ul style="list-style-type: none"> • Arthritis Australia • Asthma Australia • ausEE Inc • Australian & New Zealand Society for Geriatric Medicine (ANZSGM) • Australian Patient Advocacy Alliance (APAA) • Australian Patients Association (APA) • Australian Rheumatology Association (ARA) • Breast Cancer Network Australia • Cancer Council Australia • Canteen • Consumer Health Forum • Cystic Fibrosis Australia • The CF Pipeline • DEBRA Australia • Dementia Australia • Diabetes Australia • Eczema Support Australia • Genetic Alliance • Genetic Support Network of Victoria (GSNV) • Haemophilia Foundation Australia • Immunisation Coalition • Leukaemia Foundation • Lung Foundation Australia (LFA) • Lymphoma Australia • Melanoma Patients Australia • Melanoma & Skin Cancer Advocacy Network (MSCAN) • Multiple Sclerosis (MS) Australia • Musculoskeletal Australia • Myasthenia Alliance Australia (MAA) • National Aboriginal Community Controlled Health Organisation (NACCHO) • Neurological Alliance Australia (NAA) • Ovarian Cancer Australia • Painaustralia • Pink Hope • Rare Cancers Australia • Royal Australian College of General Practitioners (RACGP) • Michele S
Industry (pharma & devices)	<ul style="list-style-type: none"> • AbbVie • Alexion AstraZeneca Rare Disease • American Chamber of Commerce in Australia • Amgen • Antengene (Aus) Pty Ltd • AstraZeneca • Australian Pathology • Bayer Australia Ltd • Biogen Australia Pty Ltd • Boehringer Ingelheim • Bristol-Myers Squibb Australia • Camurus Australia • CSL Ltd • Eli Lilly Australia • Generic and Biosimilar Medicines Association (GBMA)

Stakeholder group	List of stakeholders
	<ul style="list-style-type: none"> • German-Australian Chamber of Industry and Commerce (AHK) • Gilead Sciences Pty Ltd • GSK Australia • Illumina • Janssen-Cilag • Lundbeck • Medicines Australia • Medtronic Australasia Pty Ltd • MSD Australia • Novartis Pharmaceuticals Australia Pty Ltd • Pathology Technology Australia • Pfizer Australia • Pharmaceutical Research and Manufacturers of America (PhRMA) • Private Healthcare Australia (PHA) • Roche • Sanofi • Specialised Therapeutics • Takeda Pharmaceuticals Australia Pty Ltd • Telix Pharmaceuticals • THEMA Consulting • UCB • Vertex Pharmaceuticals
University sector / Research	<ul style="list-style-type: none"> • Australasian Leukaemia & Lymphoma Group (ALLG) • Australian Genomics • Biointelect Pty Ltd • Cancer Health Services Research Unit, Centre for Health Policy, University of Melbourne • Cancer Research Program, School of Public Health and Preventive Medicine, Monash University • Eversana Australia • Evohealth • Health Economics and Health Services research Group, Flinders University • HTANALYSTS • Molecular and Integrative Cystic Fibrosis Research Centre, University of New South Wales (UNSW) • NHMRC Clinical Trial Centre, University of Sydney • PRIMCAT Consumer Panel, University of Melbourne • Pulse Economics Consulting • The Royal Australian and New Zealand College of Radiologists (RANZCR) • Sub-Faculty of Clinical and Molecular Medicine, Monash University • School of Clinical Medicine, University of New South Wales (UNSW)
Peak body	<ul style="list-style-type: none"> • Australian Medical Association (AMA) • Cancer Nurses Society of Australia • Clinical Oncology Society of Australia • Consumers Health Forum of Australia (CHF) • Council of Australian Therapeutic Advisory Groups • Medical Oncology Group of Australia • Muscular Dystrophy Foundation Australia • Private Cancer Physicians of Australia • Public Pathology Australia (PPA) • Rare Voices Australia (RVA) • Research Australia • Royal College of Pathologists of Australasia • Society of Hospital Pharmacists of Australia (SHPA)

Stakeholder group	List of stakeholders
Jurisdictions	<ul style="list-style-type: none"> • Independent Health and Aged Care Pricing Authority (IHACPA) • NSW Health • NT Health • Queensland Health • SA Health • Tasmanian Department of Health
Other	<ul style="list-style-type: none"> • ANDHealth • The Australasian College of Dermatologists • Black Dog Institute • Deborah Robins (Independent health consumer/advocate) • MTPConnect • Myeloma Australia's Medical and Scientific Advisory Group (MSAG) • National Paediatric Medicines Forum • Precision Haematology

Appendix 3: Written Submission Extracts (summarised by stakeholder group)

Patient or Consumer Organisations Extracts (n=34)

Consultation prompt	Inputs extracted to inform themes
Elements and features that are working effectively	<ul style="list-style-type: none"> HTA Consumer Evidence and Engagement Unit makes it easier for consumers to play a more active role and be more valued in the HTA process; Elements of the public consultation processes on market authorisation and subsidisation of new listings; The consumer representatives on PBAC/MSAC engage really-well with the patient community; the Conversations for Change initiative a useful forum to inform and provide feedback; consumer submissions to PBAC. Important to acknowledge that most of the new melanoma treatments have been listed on the PBS and are available to patients that need them currently and that Australia has a relatively good position in comparison to many other countries around the world; Reliable access to a range of high quality medicines for many people with asthma; specific funding scheme for Epidermolysis Bullosa (National EB Dressing Scheme); access to therapies for MS, asthma, diabetes. Use of high quality evidence from various types of research; gold standard randomised control trials have provided data for improved health treatments with minimal risks; thorough evidence base and review process; dedicated individuals within the HTA system (e.g. PBAC members, Department of Health and Aged Care staff). Telehealth/remote monitoring The Medicine Status website that enables consumers to search for and monitor the status of medicines as they progress through the Pharmaceutical Benefits Scheme (PBS) listing process is an important initiative. The National Mutual Acceptance Scheme was designed to harmonise ethics approvals for clinical trials within all jurisdictions. This has been an improvement, but more work needs to be done to achieve a truly national and all-inclusive scheme. For chronic diseases with large patient populations and relatively low-cost medicines, the current HTA processes and decision-making is straight-forward. This is particularly true where new therapies do not represent ground-breaking changes, from existing therapies e.g., anti-virals for COVID The parallel TGA and PBAC process has improved timelines. System can be flexible in meeting needs of rare diseases, but is slow in doing so. Registration and reimbursement processes are working effectively without unnecessary delay. Submission summary documents ('lay' summaries of the content of PBAC submissions, currently under trial) provided by sponsors were welcomed by consumer groups as a means of fostering consumer input.
Current or future barriers to earliest possible access	<ul style="list-style-type: none"> Insufficient information provided in the sponsor's application, which creates delays in the PBAC being able to make a decision; Delays in waiting for additional clinical and/or economic modelling data to be released by sponsor to support a PBS listing decision. Lower value placed on key data such as patient-reported outcomes, real-world evidence and Investigator-led data with rigid preference for Randomised Control Trial data. Multiple resubmission rounds following a PBAC decision not to recommend a PBS listing. Protracted price negotiations between government and sponsors; Delay between PBAC recommendation and implementation.

Consultation prompt	Inputs extracted to inform themes
	<ul style="list-style-type: none"> • Insufficient commercial incentives for sponsors to list a treatment in Australia; HTA policies and systems need to be responsive to the increase in personalised medicine and new and innovative health technologies; HTA policies and systems need not responsive to rare disease therapies, where patients often have limited or no treatment options and can be faced with a life-threatening illness where speed of treatment is paramount. For gene technologies and therapies - existing processes and evaluations are not fit for purpose. LSD program; these effective medications may be already approved internationally, but are not approved in a timely manner for those in need (results in lengthy advocacy to obtain treatment by patient support groups, resulting in a strain on personal resources and social stresses). • Burdensome administrative processes for prescribing biologic and targeted synthetic disease modifying anti-rheumatic drugs (b/tsDMARDs) • PBAC and MSAC committees were initially in sync with each other but have since been running on different schedules despite the reliance on each other with concurrent submissions. • Significant challenge to the HTA process is that the product under review for reimbursement can only be considered for the indication for which ARTG listed - limits timely, affordable access to best practice care for Australians with cancer. • Takes Australia an average of 466 days for a medicine to be funded once registered (and 442 days for cancer medicines.) In comparison, the patient access gap for UK is 156 days and Germany is 136 days. The duration of the TGA process compared to the FDA and EMA process. Standard PBAC meetings being held 3 times a year (ie limited flexibility / a medication cannot be assessed at any time) with agenda cut-off set months prior. PBAC meetings not occurring with the sponsor present. Rigid PBAC process generally making it difficult for PBAC negotiations to conclude prior to TGA approval occurring. More flexible arrangements not being possible (e.g. pipeline agreements where future medications from the same company would not need negotiations again – allowing expansions of age groups and genetic sub types). The overlap in what the different parties are reviewing – TGA & PBAC both looking at efficacy and then the PBAC and Department both looking at pricing (albeit the PBAC doing this indirectly). Referring issues from the Department back to the PBAC publicly adds complexity and confusion to the process whilst delaying access. • Barriers to the repurposing of existing drugs for new indications will continue to be a significant additional cost burden to government as drugs and treatments currently subsidised for one indication cannot easily be approved for additional indications where there is evidence of benefit. • Financial incentives for pharmaceutical companies to bring clinical trials to Australia. • Limited quality data, lack of patient input and affordability concerns and unknown risks are some elements that may be acting as a barrier to access. When patient information and perspectives are not adequately taken into account, the decision outcomes are adversely affected. I believe that current patient involvement in the HTA process is minimal due to barriers of access and participation. Many patient organisations are not equipped/trained to confidently provide input. Many are not even aware of opportunities to provide input. Small or niche patient organisations are particularly impacted. There are systemic inequities between patients/patient organisations and industry or health professional voices. • The existing structure and scope of the Prostheses List is a current barrier to earliest possible access to diabetes technology, particularly insulin pumps. • Current restrictions around the use of evidence in HTA processes are delaying access for patients. Lack of evidence to support public subsidy and/or a clear pathway or incentive for listing can have the unintended consequence of increasing 'off label' prescribing. HTA requires greater responsiveness to newer therapies. The system is fragmented. Separate HTA processes exist across all levels of the health system and across levels of government. This creates inefficiencies, inconsistent advice and delays access.

Consultation prompt	Inputs extracted to inform themes
	<ul style="list-style-type: none"> • Current PBS processes are not conducive for consideration of new technology, orphan or repurposed drugs or medications for Aboriginal and Torres Strait Islander people as a distinct and demonstrably smaller population group in a timely manner, if at all. • There has not been any consideration to establishing a face to face consumer voice / participation, as this survey is only open to those who are technological savvy - this is indeed discrimination against the very persons this would impact. • The current vaccine assessment process is slow and cumbersome, with the average time taken to get TGA approval more than 3 years. There is a clear need in Australia to accelerate the process for introducing new vaccines on to the National Immunisation Program; the 2 step approval process, being ATAGI, then PBAC, is unusual. It would be useful to look at other models around the world where vaccine recommendation processes are much faster.
Current or future barriers to equitable access	<ul style="list-style-type: none"> • Lack of Government funding and strategic focus for rare diseases; Lack of financial incentives for companies to develop and bring to market new treatments or innovative novel medical technologies, especially those that benefit patients with rare diseases. The most critical and challenging areas of patient access to appropriate medicines and medical devices in cancer, are the lack of commercial incentives for a sponsor to register or apply for reimbursement of their therapeutic product, especially for new indications; and the limited ability for non-commercial sponsors to access the evidence required to submit an application for a medicine, medical device or medical service for reimbursement. • Cost of medicines, including for co-morbidities; Funding of medicines does not always provide access. Some treatments require specialised staff to administer and this funding is not included in the PBS and in many cases is not allocated in other health funding. • Approval and reimbursement processes have not kept up with precision medicine and cell and gene therapy. • Better Global regulatory harmonisation of gene therapy definitions, terminology, GMP standards and timeframes for approval of clinical trials and marketing approvals may help to overcome some limitations (9). The COSEB initiative may help to harmonise clinical trial measures in the future but not for studies already underway nor completed • The pathways for approval and reimbursement for advanced therapeutics including gene therapies and gene modified cell therapies is currently not clear. • The evidentiary requirements for assessment of treatments for small patient populations poses an often insurmountable barrier for equitable access. Gold standard, double blind, randomised control trials simply cannot be delivered for rare populations and yet where other evidence, in the form of Phase II trials is accepted, the price is reduced which places a disincentive on the company to bring the drug to market early. This is why early/managed entry schemes are so critical for rare indications. Additional barriers in access to data particularly for those living with chronic or lifelong conditions such as metastatic cancer where clinical benefit is measured across a large (and increasing, thanks to new treatments) number of years that are often difficult to measure through trials prior to widespread uptake of the treatment • Currently some medical products and services to meet the needs of cancer patients are not funded by the Australian Government, leaving patients and their families to decide between forgoing treatment or paying significant out-of-pocket costs. This is not only a challenge for patients, but also to doctors in presenting the treatment options and the cost of different treatment options to patients and their families who may go into financial stress trying to afford recommended treatment. • Long, complex process for a small sub-group of patients to be added for an already approved medication. Over-use of the comparator medication to determine long term pricing. • Systemic inequities between patients/patient organisations and industry or health professional voices; leads to lack of timely access. Patients provide the 'why and how' that is relevant to the HTA process as patients have the lived experience and practical expertise in relation to

Consultation prompt	Inputs extracted to inform themes
	<p>decisions affecting them. Opportunities to be integral to all stages of the HTA process should be included for patient/patient organisations in each stage. From shaping the questions and investigations to providing patient experience information and in assessing the outcomes.</p> <ul style="list-style-type: none"> • Approximately 29 % of Australians live in regional, rural and remote areas. These areas include many diverse communities, and 34% of those communities are older adults. These patients frequently cannot afford to get optimum access to even standard medical care, and will face even more restricted access to new drugs and technology. • The shift to non-PBS, over-the-counter use may be significantly detrimental to accessibility, as CTG Script Co-Payment clients with a Concession benefit will go from no PBS co-payment to having to pay the over-the-counter price, plus this will forgo access to the infrastructure and support allowed through the PBS community pharmacy dispensing process (e.g. quality labelling and counselling). • Sponsor-led s19A approvals contribute to delays in PBS listing which means that patients of high need (e.g., Aboriginal and Torres Strait Islander people) may miss out on having timely, equitable access to affordable medicine. More broadly, the usual onus is on sponsor-led PBAC submissions and there are limited pathways in place for Department or other stakeholders e.g. peak bodies such as NACCHO to lead submissions – this poses a barrier to equitable access. • PBS listing is not based upon prioritisation of medication access to those most in need based upon medical criteria and/or non-medical condition (e.g., remoteness). • Limited mechanisms in place to ensure that Aboriginal and Torres Strait Islander people have preferential access to life saving or essential medications, despite evidence of the need to access these medications e.g. higher proportion of negative outcomes and/or higher prevalence of disease state as compared to the general Australian population. • Long timeframes for MSAC consideration. • Lack of clarity regarding de-listing of PBS medication process prior to PBAC advice being sought, and lack of clarity regarding options available if the Department and sponsor do not reach agreement, if Department cannot find another supplier or sponsor wants to de-list the medicine. • The lack of involvement with service providers, peak bodies and key stakeholders as part of price negotiation for items being listed on the PBS and MBS may create an unnecessary barrier to equitable access • HTA is complex (what it is, how does it work, why is it important) - health consumers do not have equitable knowledge about, or access to, the HTA process. Even for small consumer representative organisations, the time and commitment to fully engage with the HTA process can sometimes be constrained by available resources. The resources inequity issue is particularly stark when compared to the resources available to pharmaceutical companies.
Elements and features that detract from person-centeredness	<ul style="list-style-type: none"> • Lack of a consumer co-design process to ensure better consumer input to the evaluation process for PBAC and MSAC. HTA is a complex and often technical area, and many consumers and consumer organisations do not have the skills or resources to engage well in current processes. Consumers (as individuals) and consumer groups (representing cohorts of consumers) find it difficult to engage in the process as it is time consuming and therefore costly to contribute. The current system for determining cost-effectiveness diminishes the importance placed on mortality and disability by the general population. • There is a lack of user friendliness and transparency around PBAC processes. Documentation is not easy for people with a non technical background to engage with, and limited feedback is provided on the impact of consumer submissions on decisions, or the process of negotiation following a positive PBAC recommendation. Consumers are not privy to the information being considered by PBAC before they are asked to make comments/input. Until the OHTA website and forms are created in a consumer-friendly way, a huge barrier exists for patients to contribute to the HTA process which ultimately results in less patient centredness.

Consultation prompt	Inputs extracted to inform themes
	<ul style="list-style-type: none"> Legislative frameworks and commercial interests make it difficult for consumers to get strong and early access to information that enables early interaction and feedback mechanisms. Consumer groups often must rely on information provided by the sponsor company (usually with a commercial conflict of interest) to supply information and data regarding PBAC and MSAC listings. Little or no feedback on the value of the consumer contribution and whether it was helpful to the HTA Committees. HTA evaluation framework is weighted towards economic and financial parameters rather than the more difficult to measure elements such as the value of human lives; failing to see the person at the centre of the treatment and we don't consider what the absence of the drug does. Patient Reported Outcome Measures (PROMs) need to be incorporated into trial design and HTA assessment processes. For rare conditions, PROMs, need to be developed (where there is none previous), designed and validated for efficacy and accuracy. These evaluation tools could be used alongside QALY and DALY evaluations for a more rounded view of the impacts to individuals. Consumer groups often receive grants from sponsor companies with an interest in their specific disease area which can create perceived or real conflicts of interest. This must be carefully managed to avoid consumer submissions losing credibility. Once a medicine is recommended, consumers do not have a seat at the table or visibility of the negotiations between the sponsor and the Department which will determine whether/when the medicine will be reimbursed. Publication of a framework for evaluation of clinical and cost effectiveness for therapies for rare disease may be helpful. Key elements that currently detract from person centredness include timing of engagement of those with lived experience, communication around HTA processes including accessibility of information, and opportunities (or sometimes lack thereof) to hear directly from those impacted by a condition. Current consumer representatives on PBAC and MSAC, are not sufficiently representative of the typical healthcare consumer - patients (Patient with the condition; Patient able to provide a broad overview of the health system; Professional consumer representative) who provide their experience as part of the application process should be considered as a pool of potential committee representatives, with sufficient resources to be allocated to ensure that those individuals are supported and upskilled to be functional in HTA committee environments. Consider the difficulty of implementing PBS restrictions (e.g., requirements of an authority listing such as for sweat tests in CF) from both a clinical and patient perspective to assist towards a truly person-centred approach; there is also difficulty with medication access for those who are in a small sub-group (often due to a lack of trial data). Patients/consumers do not have an opportunity to contribute to the HTA until after HTA authority (MSAC) has received the application; patients often not involved in developing measures to evaluate clinical trials; QoL measures key to understanding patient experience but often not aligned to patient goals and preferences, exclude indirect benefits (carer effects, return to work, education, productivity etc) and not given weight in current MSAC evaluations; Strength of a consumer submission often relies on qualitative data (case studies or quotes), but this data is undervalued in the HTA process - HTA gives a much lesser value to the qualitative data that is patients' words. More strength is given to the voice of industry and health professional bodies with minimal attention on the information and experiences of patients and their representatives. The usual onus on sponsor led PBAC submissions and no/limited pathway in place for Department or other key stakeholders e.g. consumer peak bodies or individual consumers to lead submissions detracts from person-centredness.
Perverse incentives	<ul style="list-style-type: none"> PBS approval of medicines, and their subsequent use, is limited to the disease for which sponsor submissions are based. This approach prevents 'off-label' prescription by physicians as use of a PBS-subsidised drug for a new disease requires a new application backed by separate

Consultation prompt	Inputs extracted to inform themes
	<p>clinical trial data. An unintended consequence of this is that patients with uncommon or rare diseases, in which traditional major trials are basically impossible, have no mechanism through which to obtain PBS funded access to specialised medicine.</p> <ul style="list-style-type: none"> • PBAC papers and communications tend to be highly technical and not readily understandable to people who lack familiarity with bureaucratic and scientific processes and language. • Due to perceived rigidity/inflexibility of the HTA system, companies delay applications, waiting for other countries to approve treatments and demonstrate value in smaller markets. • Requirements for RCT evidence and the relationship between evidence and cost is such that rare indications, with small patient populations, which do not have RCT data to support listings are rejected on the basis of single arm studies, or not brought to market for assessment at all because companies know they won't be successful, or if they are, it's not at a price that makes it worthwhile. • Patient numbers being used as a feature of price negotiation incentivises the PBAC to always argue for the least possible (price) on a medication rather than considering the outcomes for those patients. This also slows down the process due to arguing over those assumptions / numbers. A stronger ability to negotiate price based on bulk discounts / cost savings to the health system would be a much better method to perform this function. • The lack of active horizon-scanning and reliance on market incentive (in oncology) means that the HTA is completely reliant on sponsors initiating assessment. This can adversely affect smaller cancer sub-type groups where need is less evident, and places significant responsibility on consumer organisation and other peak bodies to conduct horizon scanning and advocacy to both government and industry. • The complexity and sophistication required for successful applications favours applications by pharmaceutical companies and makes patient or clinician led applications difficult to succeed. The current process favours high return drugs/procedures. There is a perverse incentive for sponsors if a PBS listed medication is not available necessitating patients to access the same sponsor's medication with brand price premium.
Areas of interest	<ul style="list-style-type: none"> • Telehealth: Expansion of telehealth and remote monitoring via tools, such as wearable devices, to collect patient data and provide real-time insights, enhancing disease management and self-care. More public education campaigns and greater availability of devices would reduce hospital admissions. • Artificial Intelligence: A forum needs to be established for the evaluation of Artificial Intelligence (AI), either with existing bodies (Medical Services Advisory Committee, MSAC, being the most appropriate) or a separate but linked body. • Consumers: Involvement of consumers in the development, testing and evaluation of technologies. • For new EB therapies, payment approaches involving risk sharing may need to be considered, such as payments by instalment, pay-for-performance models, or sharing of costs between state and federal governments rather than more traditional PBS-type agreements. • Registry: a patient registry with provision for clinical trial recruitment would enable increased participation of relevant patient groups (e.g., people with early cognitive impairment or dementia) to participate in clinical trials for new medicines and technologies and access greater post-diagnosis support. • Early Access and Consumer Involvement: The Scottish Patient Group Partners work in partnership with patient groups around the country to capture their experiences. This is a supportive way that those with lived experience can engage with HTA, that also reassures those making decisions that they are well informed. In Germany, the AMNOG approach provides access to patients whilst the HTA process is conducted. This early access is beneficial to patients, although the option to withdraw the medicine during the assessment period would be less ideal. UK

Consultation prompt	Inputs extracted to inform themes
	<p>Cancer Drugs Fund allows access to treatments that show promise but there may still be an element of clinical uncertainty, whilst further data is collected.</p> <ul style="list-style-type: none"> • Value based HTA: For rare or orphan conditions, a committee of patient representatives which focusses on the health outcomes likely to be achieved by subsidising a technology should be considered. Once value-based HTA is defined, the function of such a committee would be to act as a reference committee for decisions that are particularly challenging due to the lack of an evidence base. • International Examples: <ul style="list-style-type: none"> ○ Using Trikafta (Cystic Fibrosis medication from Vertex Pharmaceuticals) as an example: UK: reimbursement negotiations concluded before the equivalent of the TGA approval, leading to immediate access. Germany: access generally provided then negotiations occur (much faster access, reimbursement negotiations then occur with real world data). France: at times has been similar to Germany (has an early access scheme). Ireland, Austria & Denmark: pipeline agreements previously reached, meaning future age groups or medications for people already on Vertex meds occurred without delay. New Zealand: Medsafe maximised access to Trikafta by following the US FDA listing, meaning those with rare types of CF could get access and a subsequent approval process was not needed (the listing in NZ is broader than the listing in Australia). ○ We are aware of models and processes in the UK and Canada, including the flexible process in Scotland with decision making modifiers for medicines for rare disease and the PACE (Patient and Clinician Engagement) process within the Scottish Medicines Consortium (SMC). ○ France: Inclusion of clinical added value, alongside clinical value, as a criterion e.g., based upon target population, impact on public health with impacts on medication pricing. NZ/Aotearoa: Additional mechanisms to support prioritised access for Aboriginal and Torres Strait Islander people with reduced PBS restrictions as compared to the general Australian population, similar to the approach taken by New Zealand's Pharmac Maori Responsiveness Strategy could be considered. e.g., under this policy the Maori epidemiology of a health condition which a drug is targeting must be documented and considered. • LFA notes that we are in the age of tailored medicine and treatment and that the accessibility of this is imperative for improved outcomes and survivorship. • Frailty: HTA should actively consider whether frail older people are likely to be major users of the drug/vaccine or technology, and the applicability of the evidence to that population. There is now US legislation that requires representative enrolment of older adults and other traditionally underrepresented populations in clinical trials. As this is implemented by the FDA and becomes available to the TGA, our HTA may be able to move from generalising findings to older people with multimorbidity and frailty, to using direct data, which we support. The impact of frailty on efficacy, safety and cost effectiveness is not well understood. There is international advocacy to define frailty in clinical trial populations using one or more objective measures, and to power studies appropriately to allow for subgroup analyses of clinical trial outcomes according to frailty. This will inform future HTA and relevance of findings to frail older people likely to use the medicines/vaccines and other health technologies • Cooperation: An appropriate degree of consultation and cooperation between regulatory bodies and sponsors is encouraged. However, necessary safeguards need to be in place to guard against "corporate capture" of regulators, as has anecdotally occurred with other regulatory frameworks in Australia. • Evidence: clearly a role for observational data in terms of post-marketing surveillance; but if it is feasible to perform a randomized control trial, then that should be the standard of evidence required (with exceptions for rare diseases).

Consultation prompt	Inputs extracted to inform themes
	<ul style="list-style-type: none"> Diabetes: emerging technologies and areas of interest for diabetes care may necessitate new approaches to HTA consideration of these products and funded access.
Other	<ul style="list-style-type: none"> Asthma Australia asks that DoHAC finds an appropriate mechanism to consider sustainability in terms of environmental impact in HTA and regulatory processes. It is important that such a mechanism does not prevent consumer access to critical medicines and services but rather results in favouring submissions for new and reviewed medicine and service listings with reduced carbon impact when there are alternative efficacious medicines and services to choose from. Associated waste and water footprints should also be considered a part of DoHAC's environmental stewardship, and recycling options should be available where necessary. DoHAC should work closely with manufacturers to minimise the environmental impact of the medicines and medical products We allocate resource to these comments and engage our community to ensure we are representing them accurately and effectively. It would be valuable to see a feedback process (<i>on the consumer comments portal</i>) embedded in HTA in Australia.

Pharmaceutical / medical technology company/ industry association (n=35)

Consultation prompt	Inputs extracted to inform themes
Elements and features that are working effectively	<ul style="list-style-type: none"> Indication-based pricing Confidential pricing and the ability to negotiate Special Pricing Arrangements The absence of defined ICER thresholds as a measure for cost-effectiveness, to reflect factors such as disease severity, high clinical need, and lack of current treatment options The flexibility in the approach to the economic analysis, including not always requiring a cost effectiveness submission; Parallel pathways with TGA registration and HTA assessment; and Funded indication that can be different to the indication included in the TGA registration for a medicine. Submission pathways approach with defined evaluation timelines for the different types of medicines. Publication of the PBAC Agenda to inform consumers of the opportunity to submit to the Pharmaceutical Benefits Advisory Committee (PBAC). Parallel process with the Therapeutic Goods Administration (TGA). Potential for rapid re-entry for medicines initially not recommended by the PBAC. The provision of feedback by the PBAC and Chair where a medicine is not recommended. Australia is increasingly referenced by other countries for pricing. It is crucial that the ability to have confidential prices is maintained, not only to meet Australian Government pricing expectations but to also allow the access of new medicines for Australian patients. Wider scope for decision-making instead of a set incremental cost-effectiveness ratio (ICER). HTA process can be flexible at times Informal discussions: the Department is open to having discussions with industry. Face-to-face PBAC hearings.

Consultation prompt	Inputs extracted to inform themes
Current or future barriers to earliest possible access	<ul style="list-style-type: none"> • Low willingness to pay and low ICER (not inline with inflation) • Low value of a life • Medicines are assessed differently to medical devices • Medical devices overpriced – not subject to same HTA process as pharmaceuticals • Medical devices have limitations on usage • Less competition – compare poorly globally re access to medicines • Need to incentivise launch of first in class technologies • Burdensome and costly PBAS submission process with no guarantee of success – large opportunity costs • Lack of flexibility of nuance in PBAC process • HTA process is long requiring multiple submissions (no guarantees) • Timely and lack of transparent post-PBAC processes • Limited ability to seek additional clinical expert opinion within the evaluation timelines • Standard frameworks applied across both common and rare diseases fail to consider unique attributes of rare disease treatments • Data collection challenges due to the small group of patients available for inclusion in clinical trials and limited comparative data for diseases that are uncommon • Most value assessment frameworks do not capture wider social and economic benefits (for example returning to work or improving the mental health of an unpaid carer) • Standard benchmarking tools (such as EQ5D) preferred by HTA bodies fail to fully capture the quality-of-life improvements offered by innovative treatments for rare and genetic conditions and the availability of such data is often limited in rare disease • Cost-effectiveness thresholds used by HTA bodies can lack the flexibility to fully reflect the real-world value of innovative medicines for rare diseases. • The approach to handling uncertainty, can lead to delays in access to highly innovative treatments • Redaction of commercial in confidence material from Public Summary Documents • The parallel process works well but could be even more effective in reducing time to access by, for example, the removal of the requirement for a TGA Delegate Overview at time of PBAC decision. • Special pricing arrangements and confidential pricing for medicines are important and should be retained. • Transparency operates well in parts of the system but should be improved to enhance predictability. • Multiple PBAC submissions are often required to achieve a recommendation • Limited dialogue between applicants, evaluators, and other stakeholders during evaluation • Suboptimal alignment with TGA milestones for parallel processing • Limited transparency and predictability of PBAC decision making & pricing methods • Value assessments that manage uncertainty by preferencing highly conservative scenarios and estimates • Comparator selection that does not reflect the therapy most likely to be replaced in clinical practice • Managed Access Framework that does not support early funded access for patients in its current form

Consultation prompt	Inputs extracted to inform themes
Current or future barriers to equitable access	<ul style="list-style-type: none"> • Australia is behind other countries with respect to access • Medicine reimbursement is much lower comparatively • ICER threshold is lower than in other countries • Delayed access because of lower prices • Cost of medical devices and emerging technologies • Significant commercial barriers (high costs) for launching new therapies for rare disease populations. • Rare disease patient populations are denied access to medicines because of barriers like costly and burdensome submission process • Need for greater transparency in the submission process • Need funding certainty • Need to be able to integrate all forms of evidence in the HTA process (too much emphasis on RCT data) • Difficulty of face-to-face contact with PBAC members during process • The PBAC tends to adopt a highly conservative stance on uncertainty. • Little to no consideration of disease severity, burden of disease, innovativeness, indirect costs and equity. • Inflexible application of the discount rate – weighting needed • Not set timing for pricing consideration • Heterogeneity in the disease characteristics in addition to disease history (duration, prior treatments) make measuring health outcomes across small patient subgroups problematic. • Harmonisation and collaboration across all stakeholders needed to enable sustainable healthcare systems and equitable access for patients. • There should be agreement from all parties that time to patient access for medicines means the time from when an innovative medical technology (including medicines, biotherapeutics and vaccines) is registered with the TGA to when it becomes available to patients on the PBS. • The Department of Health and Aged Care should introduce integrated and agreed data metrics and a comprehensive system for measuring the time to patient access, and report publicly in line with the National Medicines Policy. • Discount rate too high • Use of the lowest cost comparator (LCC) - For example, the LCC can be a product that is seldom used in practice or has different features to the new medicine, such as mode of administration. • No transparency about how equity is considered and addressed with certain groups (e/g. First Nations, disability, children etc) • There is currently no basis for the PBAC to recognise the benefits offered by a therapy which is orally administered (rather than injectable) or doesn't have specific cold chain storage requirements. • An approach of risk minimisation rather than representing true risk sharing • PBAC should calculate and then publish the opportunity cost of not adopting – or delaying the adoption of – a health technology

Consultation prompt	Inputs extracted to inform themes
Elements and features that detract from person-centeredness	<ul style="list-style-type: none"> • Treatment of all PBAS submissions the same. • High costs of PBAC submission for all Pharma companies • For specialist medicines for specialist populations a more tailored and patient-centered approach needed. • Lack of additional opportunities for enhanced consumer input including consumer hearings and additional time to provide consumer input to the PBAC. • Need greater engagement with patient advocacy groups – including managing the timing well to allow consultation • Patients and carers have unique expertise and insights, which should be at the centre of value assessment and given sufficient weight. • The Australian HTA system could aim to better and more consistently incorporate carer quality of life (QoL) into appraisals and decision-making. • Excessive burden on patients and caregivers who are already suffering from an immense disease burden. A lack of understanding of the complex HTA system and time constraints inhibit patients and their families from providing submissions to the PBAC. • PBAC includes only limited representation from people with lived experience, which undermines decision making. • Only two consumer representatives – not enough representation • Establish patient-expert groups • Use Citizenship counsels – like in NICE • Early and wide engagement from a cross-section of the community is needed; including involvement of patients through the HTA evaluation process. • Greater use of social values and patient experience to reveal the full value of the technology. • The system as it is currently designed does not consider person-centeredness • Evaluation favours traditional clinical trial and cost effectiveness methodologies. • System does not adequately value a patient's lived experience and how important quality of life is • The payer's over-emphasis on price as opposed to patient benefit/outcomes. • Need more consideration of budget impact rather than ICER • an unsustainable dynamic has evolved between the pharmaceutical industry and the PBAC in recent years, which disincentivises collaboration
Perverse incentives	<ul style="list-style-type: none"> • Assessment and funding processes for medical devices should be aligned with pharmaceuticals • The PBAC tends to adopt a highly conservative stance on uncertainty. • Choice of comparator during the HTA evaluation may lead to unintended consequences - i.e. PBAC may choose the cheapest therapeutic alternative rather than the treatment that the new medicine is most likely to replace in clinical practice which may fail to recognise and value innovation. • Pricing policies: are creating unintended consequences of innovator medicines (F1) being priced at or below generic comparators (F2) without consideration as to the mechanism of action or adverse effects of similar therapeutics. • Perception of compassionate access: The way in which compassionate access is perceived can give the false impression that there is no need for payer intervention. However, not all companies provide compassionate access and the criteria for these programs are often quite strict and inequitable. One criterion that can be required – for example- is that the medicine is due to be reimbursed in the relevant country within a particular timeframe.

Consultation prompt	Inputs extracted to inform themes
	<ul style="list-style-type: none"> Life-saving medicines are de-prioritised: The Life Saving Drugs Program requires medicines to be rejected first by PBAC before then being evaluated by the LSDP Expert Panel. Due to the protracted nature of this “double-process”, life-saving medicines for small patient populations take longer to be funded. No paediatric, orphan drug/rare disease incentive: The incentives are limited to regulatory and reimbursement fee waiver (one submission only). There are no additional incentives for disadvantaged patient sub-groups. An unintended consequence of listing new MBS items without industry consultation may mean low access levels, non-viable funding and impacts of equity for Australian health consumers.
Areas of interest	<ul style="list-style-type: none"> Horizon scanning Note: equity is linked to access (inter-related). Poorer access leads to greater inequity Need to look internationally for what is working (Germany, France and UK) Ethics of denying access
Other	<ul style="list-style-type: none"> Timely access leads to range of other benefits (societal level) can produce wide range benefits not captured - proactivity gains, reduced carer burden. Such broad benefits need to be considered.

University sector / Research (n=14)

Consultation prompt	Inputs extracted to inform themes
Elements and features that are working effectively	<ul style="list-style-type: none"> Detailed and timely evaluations of PBAC submissions by external economic evaluation groups; meetings between the Sponsor and PBAC Chair post negative recommendation. Secretariat functions are clear and communicative, and there is opportunity to engage with health technology resourcing team members at post-MSAC sub-/committee meetings; the review of economic evidence is effective and there is good transparency in outcomes, with reports developed by different review committees becoming publicly available in a timely way; members of the health technology resourcing team available to provide guidance to applicants are experienced, and knowledgeable, and greatly facilitate the application process. MSAC and PBAC engages with independent academic groups to conduct independent evaluations of submissions. This collaborative approach ensures a rigorous assessment of healthcare technologies and interventions while minimising the potential for bias assessments. By involving external experts and organisations, MSAC and PBAC has drawn upon a diverse range of perspectives, knowledge and skills, enhancing the credibility of the evaluation process. Key factors that enable these (PBS) funding decisions include: <ul style="list-style-type: none"> 1. Flexibility to work with threshold values for the incremental cost-effectiveness ratio ICER), to make some landmark approvals for truly innovative therapies to treat cystic fibrosis, spinal muscular atrophy and other rare diseases; 2. Initiating the involvement of health consumer organisations in HTA decision making to inform key issues relating to quality of life and patient experience; 3. Flexibility to incorporate value considerations outside of those specified in the PBAC guidelines, including impacts on family and carers (carer utilities); 4. The introduction of a rapid HTA processes to approve therapeutics indicated for COVID-19 during the public health emergency.

Consultation prompt	Inputs extracted to inform themes
	<ul style="list-style-type: none"> • Evidence levels expected are of high standard; mechanisms in place for fast-track decisions, although these are not consistent and not well known; PBAC transparency – agenda, minutes and summaries are quickly and readily available; appointment of independent experts with strength in areas of health priorities. • One area of positive development has been the introduction of new Therapeutic Goods Administration (TGA) pathways, which aim to expedite access to ground-breaking treatments.
Current or future barriers to earliest possible access	<ul style="list-style-type: none"> • Difficulty in defining an acceptable PICO a-priori; rigidity of evaluation timelines and process (inability to submit new data). There is currently not enough flexibility in the HTA process to recognise clinical best practice that does not align with the extensive evidence base currently required. • Necessity to undertake large studies to accrue sufficient evidence of the "magnitude of benefit" and uncertainty in applying this evidence to heterogeneous populations. Requirement of randomised control trial data to support approvals process • Insufficient coordination between HTA committees to provide a person-centered model of care in which reviews and recommendations are synchronized and streamlined, for example coupling newborn screening and pre-symptomatic treatment of SMA together to enable better outcomes; an additional barrier to earliest possible access is not enough support post HTA approval for healthcare practices to deliver complex therapies. • From a consumer engagement standpoint, the voice of the consumer is under-represented in the Australian HTA system; there is no pathway or transparent system that would encourage the participation of consumers to give feedback or have input into Health Technology Assessment processes. • Lack of agility and lengthy timelines associated with usual review pathways including HTA processes. As per Recommendation 29 of the New Frontier report - the inflexibility of HTA and how they are not fit for purpose for new technologies, drugs, and other precision medicine approaches; As per Recommendation 23 of New Frontier - lack of a national register of clinical trials associated data and rare and genetic disorder registries will hinder the development and collection of evidence required for novel therapies • Without a readily identifiable process (to fund technologies via the AHRA/IHACPA), and little engagement with the applicants, the case for funding lacked momentum and progress. • There is little opportunity for knowledge exchange and shared decision making between the applicant, MSAC and advisory groups; there is no mechanism for formal acknowledgement of experts who have supported HTA applications or assessments • Lack of formal systematic horizon scanning program or early detection system to support HTA and Sponsors in determining evidence requirements to facilitate successful submissions. • Lack of guidance (and acceptance) on real-world evidence (RWE) integration. A consistent approach to the use of RWE would improve its integrity and reliability and provides HTA bodies with valuable insight into patient outcomes outside of clinical trials. • Enhancing the capacity to collect and utilise RWE in a more efficient and expedited manner will provide a more comprehensive assessment of treatments' real-world impact and enable earlier access to innovative therapies. • Lack of data linkage on a national scale and inconsistent approaches to data linkage within the Australian states and territories. • Substantial time delays between the initial positive recommendation from PBAC or MSAC and the actual listing of a healthcare technology. Federal approval however state implementation delayed, deferred, or altered. • Current HTA guidelines inadequately address the technical, methodological, and practical challenges of evaluating clinical genomics applications.

Consultation prompt	Inputs extracted to inform themes
	<ul style="list-style-type: none"> • Vaccine assessment processes are long, costly and inefficient, with multiple reviews and decision-making • Government is less willing to pay to prevent disease with a vaccine than it is to treat disease. PBAC valuation methodologies can impact the ability for vaccines to launch effectively in Australia. The Incremental cost effectiveness ratio (ICER) ('value for money') threshold (\$15,000) for vaccines is well below that of other medicines • Discount rates used in economic evaluations favour investments in interventions that deliver short-term vs. longer term benefits, such as vaccines and gene therapies. • The current processes help drive down costs of drugs and devices to the Commonwealth and the taxpayer. However, they do so at the expense of very substantial delays in access for persons suffering ill-health and in need of such interventions at the present. If the patient doesn't need the new test or new treatment for 2-3 years, then the system is fine. • Unclear how adoption or consideration to international peer review or comparisons are made • Inconsistent data collection of product in use (is a) challenge to understand equitable or timely access • The use of 'current standard of care' as a cost comparator for new medicines is a harsh and unrealistic bar to set for diseases in which there is no approved standard of care. ... For cost comparison to be made against off label off patient ineffective unapproved standard of care is simply wrong, and as a result Australian patients are suffering. • Decision-making processes often prioritise a cautious approach, particularly when it comes to adopting innovative technologies. In practice this means evaluators will select the most conservative estimate of treatment effect, rather than the most likely. • Although a MEA pathway exists in Australia, uptake and use by Sponsors/Decision Makers has been poor. • The HTA system's narrow definition of value and impact poses another barrier to timely access. Traditional measures, such as cost-effectiveness and clinical efficacy, often take precedence, disregarding broader aspects of patient well-being and societal impact. By expanding the evaluation criteria to include patient-reported outcomes, quality of life improvements, and the broader socio-economic benefits of innovative therapies, the HTA system can better reflect the true value and potential impact of these interventions, allowing for more efficient access. • Lack of Vision around the Value and Importance of Genomics - essential to establish an appropriate evaluation framework that considers the evolving nature of genomic testing, interpretation, and targeted therapies. • Lack of a Fit-for-Purpose Process for Rare Disease Therapies: the MSAC pathway imposes a standardised, "one size fits all" evaluation process which doesn't account for different technology environments. • Lack of Funding Certainty for Cell and Gene Therapies: establishing a more transparent and predictable funding process specifically tailored to these therapies is essential to ensure timely access for patients in need. • Lack of Public Input and Transparency: The HTA process being conducted behind closed doors without meaningful public input can act as a barrier to the earliest possible access. • The HTA process is complex and lengthy, and it can be difficult for some clinical applicants to be able to dedicate the time required to an application process; commercial organisations have a greater pool of resources to complete applications compared to not-for-profit organisations such as Medical Colleges or individual clinicians.

Consultation prompt	Inputs extracted to inform themes
Current or future barriers to equitable access	<ul style="list-style-type: none"> • Protracted process has created inequities and divided the SMA community (stakeholders have expressed guilt, feeling abandoned and not valued). Prior to HTA approvals, people with SMA have been seeking treatments via participation in a lottery, travel overseas and fund-raising campaigns (Go fund me). • The current HTA process is not responsive to patients and their needs. • There is no support with post-MSAC approval education of medical professionals, which likely means that some professionals are not accessing tests that would be beneficial for their patients because they are unaware of their availability or significance to their patient. Further, MSAC's approaches to which medical professional can order tests; the rebate amount, and for genomic tests whether there is gene list, or no gene list are all areas where inequity could be introduced, in terms of patient access, timeliness of result and results reported. • Insufficient consideration of equity implications in cost-effectiveness and budget impact analysis. • Until HTA processes prioritise the needs of Australians who are currently unwell rather than those diagnosed with cancer sometime in the future, we are limited to a model that treats the patients of today as second-class citizens. • Inequitable because as soon as drugs become available on the market anywhere in the world – in this hypothetical example, immediately after FDA approval – wealthy Australians can and do access these treatments from international markets. • It is clear that the committees understand the problem (<i>of demonstrating efficacy in rare diseases with small populations</i>) but the current structure does not allow for new technologies or assessment of efficacy using novel trial designs such as n-of-1 and adaptive trials.
Elements and features pertaining to person-centeredness	<ul style="list-style-type: none"> • It would be helpful to define person-centredness to ensure consumers understand what the HTA Review process means by this term. • Co-design and partnership are important aspects of the system - consumers must have equal power/weight in the creation, operation and evaluation of HTA systems. • Extent of stakeholder input or influence in HTA decisions is unclear, and there is insufficient funding support for consumer-driven submission applications; Medicines are increasingly being approved based on earlier trial data which includes an over-reliance on surrogate endpoints. However, it is unclear if these endpoints accurately reflect consumer priorities. • HTA processes are meant to be patient-centric, but no priority seems to be given to existing patients - patients requiring treatment today and tomorrow and the day after. • The policy (at PBS) allowing aged cap access can detract from person-centeredness. A lack of funding for tests through the MBS limits access to diagnostic tests that are identified to be standard of care by prevailing international clinical disease-guidelines. • Quoting Zimmerman: '[there is] limited timeframe and quick turnaround', which it said showed 'the inadequacy of PBAC's current mechanisms to seek consumer input.' and 'it is difficult to engage with a PBAC process when there is insufficient information provided from PBAC' and 'consumers bring a crucial lived experience' and 'processes for engagement need to be both meaningful, transparent and have a genuine impact/weighting in the decisions.' The report emphasised the need for feedback to be provided to patients 'to facilitate continuous improvement in the contributions made, methods are needed to incorporate data and evidence provided by patients' into HTA' and 'HTA systems need mechanisms to incorporate data and evidence provided by patients' • A 'health budget only' perspective fails to recognise the full value that the medicine provides to the patient and/or their carers. • Nonetheless, based on evidence and comments by patient groups to the NMP and HoR inquiry reviews, there seems to be a perception that some HTA assessors tend to consider patient experiences lower in the evidence hierarchy and thus have limited impetus to integrate this type of evidence in the evaluation process. Use of objective tools (patient reported experience measures (PREMs) and patient reported outcome measures (PROMs) to measure patient experience/outcomes should address these potential concerns by HTA bodies. By incorporating PREMs into the

Consultation prompt	Inputs extracted to inform themes
	<p>evaluation of a medicine, HTA bodies can gain insights into how well the treatment aligns with patients' needs, expectations, and preferences. By including PROMs in HTA assessments, decision-makers can better understand the tangible benefits and potential trade-offs associated with a particular medicine.</p> <ul style="list-style-type: none"> • Outcomes that deny Australian patients access to breakthrough medicine have direct person-centered harmful effects. • By involving patients throughout the evaluation process, we can ensure that their unique perspectives and needs are fully considered, leading to more patient-centric outcomes. In addition, it is crucial to raise awareness and involve the broader community in the HTA process. The HTA system should engage with the broader community to ensure a more inclusive and representative decision-making process. • Lack of Flexibility and Evolution: The HTA system currently lacks the necessary flexibility, particularly when it comes to rare diseases that do not fit within the existing frameworks such as the Life Saving Drugs Program (LSDP). • The Department does not take a proactive approach (to patient inclusion), and only reaches out to key consumer groups or colleges/clinician groups. However, the individual patient, who is essentially the end consumer, may be completely unaware of these groups or the opportunity to lend their voice.
Perverse incentives	<ul style="list-style-type: none"> • Price signalling - sponsors inflate initial price to have 'room to move'; PBAC rejects on basis they know initial price is higher than needs be. • A particular area of focus may be “superseded care” - when new and improved treatments are approved, are old treatments removed? Are there some patients who will not receive access to the latest treatments based on lag in clinician uptake? How does the HTA system monitor for and manage such risks and issues? • Choice of Comparator in HTA Evaluation: The current practice of selecting the cheapest alternative as the comparator during HTA evaluations can have unintended consequences. This approach fails to recognise the value of innovation and discourages submissions from companies. • Insufficient consideration of individual-based models in current HTA guidelines for cost-effectiveness analysis, especially in clinical genomics applications; HTA policies and methods that focus primarily on short-term outcomes or fail to adequately consider the long-term benefits and broader value of health technologies may create unintended consequences. • The issue of RSA's is that risk is not shared, but shifted from the payer (Commonwealth) to Manufacturer. For products which have significant benefit, but have immature data, RSAs provide a disincentive to list in Australia and reduces the priority of the Australian healthcare market to commercialise a therapy. In addition, access for patients is reduced.
Areas for further investigation	<ul style="list-style-type: none"> • Care needs to be taken when duplicating models from other countries which may have different health systems and needs. Consideration of unintended consequences is an important factor in evaluating external models. Co-design and the consumer voice is imperative in evaluating these models to ensure unintended consequences are considered, from a patient perspective. • The UK's National Health Service process for adding new tests to the national genomic test directory appears to be an efficient, high-throughput process that engages a panel of experts to consider new genomic tests. Elements of this process should be investigated for consideration for adoption in Australia. • The following was identified as elements of the HTA policy, method, mechanism suggested for consideration from NICE: <ul style="list-style-type: none"> ○ 1. Comprehensive guidelines: Technical support documents by NICE Decision Support Unit (through the University of Sheffield) provide clear and detailed instructions on various approaches for HTA assessments; 2. Broad stakeholder engagement: NICE encourages active involvement of a wide range of stakeholders, including consumers, healthcare professionals, industry representatives, and relevant experts, throughout the HTA process; 3. Consideration of broad societal impacts: NICE expands the scope of the HTA evaluation to include the

Consultation prompt	Inputs extracted to inform themes
	<p>assessment of broader societal impacts beyond clinical effectiveness and cost-effectiveness. This includes considering productivity gains, social value, and the impact on caregivers.</p> <ul style="list-style-type: none"> The following was identified as elements of the HTA policy, method, mechanism suggested for consideration from CADTH: <ul style="list-style-type: none"> 1. Consumer involvement: The CADTH involves consumers and consumer groups throughout the HTA process by various means such as consumer input submissions and a consumer group input forum. This ensures that the consumer perspectives and preferences are considered during evaluations. The HTA Review should consider incremental steps that can be taken towards improving access, conducting better studies and, ultimately, making better use of these (post-marketing) studies in HTA decision making. UK Cancer Drugs Fund In the UK, the NHS has an Independent Request for Funding pathway for patients to access repurposed medications https://www.england.nhs.uk/contact-us/privacy-notice/how-we-use-your-information/our-services/individual-requests-for-funding/ New Zealand has 2 pathways to address inequity in access: <ul style="list-style-type: none"> One is the Named Patient Program intended for orphan conditions, such as cystic fibrosis or exceptional condition criteria - https://pharmac.govt.nz/medicine-funding-and-supply/make-an-application/nppa-applications/ ; The second pathway is a special waiver scheme for drugs that do not meet all the criteria but the physician believes they fulfill the spirit of the special authority - https://pharmac.govt.nz/medicine-funding-and-supply/make-an-application/special-authority-waiver/ If interested in novel approaches to pricing: <ul style="list-style-type: none"> Danny Palnoch (Head of Medicines Analysis – Commercial Medicines Directorate) would be the best person at the NHS to speak to in regards to innovation in pricing at the NHS (danny.palnoch@nhs.net).
Other	<ul style="list-style-type: none"> There are several important considerations and challenges for implementing HTA recommendations for rare diseases : <ul style="list-style-type: none"> Clinical referral networks and educational resources need to be established and accessible to provide diagnostic and sustainable treatment services and co-ordinate follow up for people and families with rare diseases; These require expertise in medical genetics, neurology, and the provision of advanced therapies for rare diseases; A coordinated strategy to collect, measure, build and translate data is needed to support quality improvement activities, monitoring of treatments and long-term outcomes, revision of clinical care guidelines. HTA (Review) should consider: <ul style="list-style-type: none"> Commission and fund clinical trials in rare diseases that involve repurposing of old drugs; For new therapies or technologies that have insufficient evidence to support reimbursement in the Australian market, enable a pathway whereby the PBAC or MSAC could commission research with the new agent only available to be studied in the context of a clinical trial or registry in order to collect data to support reimbursement. (Lack of) Access to clinical trials is a major barrier to patients being able to access new procedures, tests, and medications. If international work sharing was used, it would only be relevant for Section 2 of a PBAC or MSAC assessment, as the clinical pathway/treatment algorithm may differ, and costs and resources may also differ, requiring complete reassessment of Section 3 and 4.

Peak body (n=9)

Consultation prompt	Inputs extracted to inform themes
Elements and features that are working effectively	<ul style="list-style-type: none"> • Australian HTA policies and methods are appropriately rigorous; key committee personnel demonstrate requisite expertise and a comprehensive understanding of relevant issues. • (MSAC's) acceptance of clinical effectiveness evidence from international jurisdictions demonstrates an acceptance of clinical best practice whilst at the same time reducing duplication of effort and reducing assessment costs. • Current HTA policy and methods have (at times) considered the broader implications of a health technology on the health system (e.g., highly specialised therapies and their associated ancillary services are jointly funded by the Australian Government and all states and territories under the National Health Reform Agreement (NHRA), supporting national access to treatment for patients with rare conditions). • Strong involvement of medical practitioners in HTA decision-making processes is strongly supported. • Improved consultation with peak bodies, leading to better decision-making. • The Office of Health Technology Assessment (OHTA) Consultation Hub is effective and provides useful guidance to consumers and consumer organisations in providing comments. • Processes and time frames for making consumer comments is transparent. • Concurrent processes for regulatory and reimbursement approval potentially improve timeliness of access. • Expedited resubmission pathways have reduced some of the impact resubmissions have time to access for patients. • The Consumer Evidence and Engagement Unit provides a effective pathway for small, under-resourced and inexperienced patient groups to engage with HTA processes. Consumer comments have been used to address areas of uncertainty in submissions for rare disease therapies, particularly in providing evidence of patients' lived experience, quality of life, reduced treatment burden and reduced disease burden. • Acknowledgment of the 'value of knowing' by the MSAC. Not all diagnostic tests result in an actionable treatment, but the value of knowing may enable the end of lengthy diagnostic odysseys, especially for rare diseases. • Acceptance of diagnostic yield as an appropriate measure of test performance. • Reduced emphasis on gene lists in item numbers, acknowledging that the evidence base in genomics is rapidly evolving, as are the methodologies used to detect pathogenic variants.
Current or future barriers to earliest possible access	<ul style="list-style-type: none"> • It is not clear whether purported delays in listing are inherently harmful to consumers, or whether they represent the system working with appropriate checks and balances. Timely approval processes and access to new technologies must not come at the expense of consumer safety. • The rigid structures of the current HTA process cause delays to patient access (e.g., Shingrix was not receive a PBS listing for more than a year after evidence to support its subsidy had been available). • Early access can be expedited by industry through 'Medicine Access Programs'. • Lack of coordination between Government/s. The Australian Government's system for providing access to health technologies is complex and inefficient; a significant amount of overlap leads to delays in access (e.g., safety is assessed by the TGA, PBAC/MSAC and Drug Therapeutic Committees responsible for the governance of the medicines management system in health service organisations. Clinical effectiveness is assessed by PBAC/MSAC and DTCs). • Applications for emerging technologies, particularly pharmacogenomic technologies, are often highly complex and may require HTA evaluators to have a greater level of specialist knowledge. • Lack of consultation with the acute care sector during the HTA process—current HTA process does not allow for sufficient consultation with stakeholders in the acute care sector who have a significant role in the funding, delivery, prescribing, supplying, and administration of these health

Consultation prompt	Inputs extracted to inform themes
	<p>technologies, which may vary substantially from the primary care setting. Much of hospital prescribing pertains to off-label use (particularly of generics, as prohibitive costs of HTA and anticipated low-revenue discourages sponsors for submitting drugs for new indications; e.g., Domperidone for off-label treatment of lactation insufficiency) and Special Access Scheme medicines, which are poorly reflected in current HTA evaluation processes.</p> <ul style="list-style-type: none"> • Sponsors seeking to apply for new or expanded indications face significant regulatory burdens. The cost and complexity associated with submitting (often multiple) applications for assessment of new health technologies and/or indications by sponsors is currently a significant barrier to early access to new therapies, especially in the areas of rare disease and for therapies targeting children. Time and costs associated with submissions are also a significant barrier to submissions for generic medicines. • MSAC/PBAC application processes are burdensome, lengthy, expensive and ill-suited for many emerging health technologies. In the case of pharmacogenomics, applications must be submitted by variant or indication, which is time consuming and does not keep pace with this rapidly developing technology. Applications should not be specific to particular pathology platforms/analysers, as this locks pathology providers into contracting with certain suppliers with whom they may have little control over platform and consumable costs. It can also mean that laboratories need multiple platforms for similar tests (e.g., immunotherapy assay PBL1 - testing many antibodies on specific platforms). The capital intensive requirement for specific equipment is a disincentive to provide these items. It also increases workload and risk of errors. Laboratories need flexibility to choose the appropriate platforms for their services. • Prioritisation of evidence from randomised controlled trials compromises a barrier to timely, equitable access to rare disease health technologies; in some cases, this may discourage companies from submitting therapies for particular indications (e.g., The sponsor of Miglustat for patients with Niemann-Pick Type C has decided not to pursue reimbursement via PBAC/LSDP due to a lack of evidence, creating uncertainty for patients currently accessing this therapy via hospital funding/compassionate access). • Appropriate comparators may not exist for emerging technologies; as a result, the referenced fee of a nominated comparator may not reflect the costs of the new technology. • The Pathology Services Table of the MBS (PST) has not been updated to reflect contemporary clinical practice nor the current cost of pathology tests. Sponsors often misrepresent the true costs of pathology services in HTA applications. • HTA processes could be made more efficient through enhanced synergy between MSAC and PBAC processes for pharmacogenomic applications. • The need for a technology to have completed the PBAC process prior to referral to the Life Saving Drugs Program (LSDP) delays access for rare disease patients. The LSDP approach should be incorporated within PBAC to eliminate the need for a secondary/dual process. • Managed access programs should be used to address uncertainties, including through the collection of real world data. • PBAC/MSAC information requirements are not specific enough, leading sponsors to provide unnecessary data. • The acute care sector is often unprepared to deliver certain high-cost therapies; it may take years before a medicine listed on the PBS is added to a hospital formulary and available to patients in the acute care setting. As part of the assessment process, HTAs should undertake an impact assessment reviewing the capacity of the healthcare system, particularly the hospital sector, to deliver the health technology being assessed. • Horizon scanning—There is a need for a nationally coordinated and systematic process of identifying and monitoring emerging technologies that have the potential to significantly impact the acute care sector. • Australia must seek to increase its capacity for clinical trials by funding more research and improving the clinical trials workforce in Australia. • Suitable funding pathways are required to ensure hospitals' ability to deliver precision medicines. • Sponsors are frequently unable to ensure supply of approved medicines, leading to shortages in acute settings.

Consultation prompt	Inputs extracted to inform themes
Current or future barriers to equitable access	<ul style="list-style-type: none"> • Similar products should be assessed transparently according to consistent standards of evidence. • Due to the lack of integration of HTA processes between the states/Commonwealth, technologies funded through mechanisms other than PBS/MBS (hospital funding/compassionate access/pharma funded/self-funded) may be delivered inconsistently or inequitably, with patients in some jurisdictions and hospitals able to access treatments while others cannot. • HTA processes could be made more equitable through alignment and clarification of MSAC and PBAC assessment pathways for therapies that do not fit neatly into traditional categories (e.g., emicizumab is classified as a medicine in the regulatory arm (TGA) and as a blood product from a funding perspective (MSAC). Opting for evaluation via MSAC may comprise an effort to cost-shift as MBS items may be subject to cost-sharing provisions via the NHRA. • The parallel (TGA/PBAC) process can introduce uncertainty when a therapy is approved for subsidy but has not had sufficient data generated needed by prescribers to evaluate proper use and cost-implications. • Disconnect between PBAC and MSAC HTA processes sometimes result in pharmaceuticals being listed on the PBS without their co-dependent pathology test being listed on the MBS; these tests may not be conducted or may be undertaken at the expense of patients, creating barriers to equitable access. • In the case of identified equity groups and rare diseases, limited clinical data should not be used as a lever in price negotiations. Aggressive negotiation on price discourages international sponsors from engaging in the relatively small Australian market. • Individual Patient Usage (IPU) data held by hospitals and hospital networks are an untapped resource of independent clinical evidence and information to assist with the identification of medications/indications in Australia. There is currently no avenue for hospitals to provide advice to DoHAC/PBAC on medicine use and benefit in the acute setting. • Processes should be formalised to enhance consumer engagement in TGA regulatory approval. • Reliance on commercial sponsors may lead to delayed/inequitable access to therapies where there is insufficient commercial interest. • Jurisdictional DTCs do not have the necessary resources or access to the evaluation data needed to complete detailed cost effectiveness analyses. The sharing of Commonwealth commissioned HTAs with jurisdictions would improve the judicious, appropriate, safe, effective and cost-effective use of medicines nationally. Moreover a collaborative cross-jurisdictional approach to HTA would foster streamlined and more consistent decision-making, increase equity of access to medicines nationally due to the availability of expert assessments, reduce lost opportunity costs and decrease duplication of effort undertaken by individual states, healthcare services and hospitals. • Current hospital funding arrangements do not reflect the emerging medicines landscape, which increasingly requires acute and in-patient hospital administration. New funding models (i.e., national single-payer) and reimbursement pathways are needed.
Elements and features pertaining to person-centeredness	<ul style="list-style-type: none"> • PBS restrictions for some health technologies are approved by PBAC under specific clinical guidelines more commonly relevant to treatment in the primary care setting; PBS prescribing restrictions may not match clinical guidelines or appropriately consider the sustainable use of these medicines in hospital and acute settings (e.g., Shingrix, a vaccination for shingles, is subsidised for bone marrow transplant patients but not other transplant patients; PBAC recently increased the fluticasone Junior inhaler's general listing to an authority listing (requiring a specialist prescriber) for patients under 6, which introduced unnecessary barriers to accessing safe and recommended care. • PBAC has a role in making recommendations to Government to subsidise alternative medicines where known shortages compromise patient access to affordable medicines (e.g., Maxolon for the treatment of nausea and vomiting in patients with cancer and in palliative care is the only brand of metoclopramide hydrochloride monohydrate listed on the PBS, despite the availability of two other brands).

Consultation prompt	Inputs extracted to inform themes
	<ul style="list-style-type: none"> HTA processes do not adequately reflect contemporary and emerging models of care (i.e., the patient journey is no longer a simple pathway back and forth between hospital and community settings). Individual utility should be given greater weight in the assessment of therapies for rare diseases, where other evidence types are less available due to sample size, etc. Clinical trial design is often not person-centred. The value of knowing should be applied as a principle in all HTA policies and methods. Data collection is not sufficiently systematised and coordinated to enhance person-centred care, including collection and analysis of data on off-label use, including in acute and rare disease settings, non-PBS medicines and adverse events. Australia should emulate HTA processes in the European Union (e.g., EUPATI) that invest in the training of patient advocates to work alongside clinicians and enhance patient engagement throughout the HTA process. Consumer engagement in HTA processes must be early, co-designed with consumers, targeted, routine, proactive, inclusive, reciprocal; iterative, formative and outcomes-focused. Decisions on access (including TGA determinations on indications) should not be made without genuine consumer engagement. It is not currently possible to gauge the effectiveness and/or impact of consumer engagement in HTA processes. Changes to policy/methods legislation are required to give consumer evidence greater weight in cases where small patient population numbers mean randomised controlled trials (RCT) data is limited/underpowered. Consumer organisations require education and feedback to understand the types of evidence that are applicable in the HTA setting and how they can best collect this evidence, including feedback on improving the quality and effectiveness of the comments they provide. Consumer engagement could be enhanced by providing additional information on HTA process and timelines. The Consumer Evidence and Engagement Unit should be appropriately resourced to increase public awareness and improve utilisation of the unit. MSAC should be consulted to facilitate consumer engagement in the consultation survey in a similar way to PBAC consumer comments. PBAC HTA processes should incorporate input from states (and hospitals) as is possible under MSAC processes. The Closing the Gap PBS co-payment program for Aboriginal and Torres Strait Islander people excludes medicines dispensed at discharge from public hospitals.
Perverse incentives	<ul style="list-style-type: none"> There is potential to cost shift between National Health Reform Agreement-funded services and MBS-funded services depending on whether technologies are listed on the PBS/MBS and given differing payment rates for providers under the MBS (e.g., higher MBS fees to private pathology providers compared to public pathology providers for every pathology episode). The lack of access to PBS subsidised medicines for public hospital inpatients results in cost shifting. Incentive to claim for an episode of care each time a patient attends an outpatient appointment can at times be a perverse incentive to increase the frequency that that patient must present to receive treatment, although it may not always be necessary.
Areas for further investigation	<ul style="list-style-type: none"> There is a need to expand the concept of 'value' in HTA and for the TGA and, PBAC and MSAC to examine the clinical, social, and financial value of approving or subsidising a health technology to enable access to patients requiring it, compared to not approving or subsidising it (i.e., what are the implications of disease progression on a range of factors including, mental health, family life, loss of work, and hospitalisation). Traditional boundaries between medicines, medical devices and personal products are being blurred; HTA processes must be updated to avoid arbitrary siloing and delimitation of technologies that do not fit neatly into old categories (e.g., pharmacogenetic therapies, stem cell therapies).

Consultation prompt	Inputs extracted to inform themes
Other	<ul style="list-style-type: none"> • A process for disinvestment in health technologies that are no longer fit-for-purpose should be formalised, with PBAC and MSAC empowered (and sufficiently resourced) to provide relevant guidance to Government on these matters. • The DoHAC process for stakeholder consultation, particularly the language used in the Terms of Reference to guide submissions, is too complex and opaque to effectively engage and solicit the views of consumers. Ideally such a process should be co-designed with consumers from the ground up and be part of a longer-term investment in community engagement in national medicines policy. • Evaluation of rare disease technologies must be fit-for-purpose and account for complex delivery in partnership with state health systems. System levers to improve timely and equitable access to rare disease health technologies include: committee oversight of managed access arrangements; centralised collection of real-world data; differential discounting; addressing uncertainty through use of alternative forms of evidence (e.g., personal utility, value of knowing, consumer evidence); incorporation of rare-disease expertise in decision making; eliminating dual processes where possible (e.g., PBAC/LSDP); use of National Hospital Funding Agreement to ensure alignment of access across jurisdictions). • A lack of funding from NHMRC and MRFF to research and develop HTA methods means many Australian evaluators find it challenging to progress through traditional university career pathways. Many evaluators move over to the pharmaceutical sector where wages are higher and career paths are clearer, putting pressure on the Department of Health and Aged Care and evaluator teams.

Jurisdictions (n=6)

Consultation prompt	Inputs extracted to inform themes
Effective elements/features	<ul style="list-style-type: none"> • Detailed and appropriate analysis of safety, effectiveness and cost-effectiveness (across all HTA agencies and pathways). Their assessments include consideration by skilled independent multi-disciplinary committees, and published advice. • The current HTA process has been effective at ensuring safety and quality of medicines for Australian consumers. It is acknowledged that recent changes to HTA internal business practises, have helped to reduce length of time to HTA access. • Current process to determine the assessing entity for Highly Specialised Therapies (HSTs) under the National Health Reform Agreement 2020-2025 (NHRA) is considered very effective. This includes joint decision making by the Joint Chairs (PBAC, MSAC and the State and Territory representative) to identify the appropriate HTA pathway. S&Ts being engaged and informed at an early stage by the Commonwealth and able to provide comment to the MSAC
Current/future barriers to early access	<ul style="list-style-type: none"> • Early approval and funded access to certain therapies (HST e.g., CAR-T) in the absence of high-quality evidence may place patients at risk - risks to patients of early access do need to be considered in the HTA and approval process. Early access does not always represent safe, effective or cost-effective access. There needs to be acknowledgement that timely access needs to be counterbalanced with robust post-HTA reviews, utilisation and mechanisms. • Submission-based assessments provided to the MSAC and PBAC by the sponsor remain commercial-in-confidence and are unpublished which limits external review and transparency. • PBS restrictions may not stay consistent with evolving clinical practice or clinical guidelines. • Although Australia has approved the funding of novel HSTs of NHRA in recent years, there remains a lack of a formally recognised approach to coverage with evidence development (CED) for these therapies. Depending on the therapy, it may not be possible to demonstrate long term efficacy and safety until years of follow-up is completed (e.g., gene therapies). Agreed timelines for re-evaluation processes should be articulated in the HTA to manage this uncertainty, and lack of real-world efficacy should inform disinvestment as appropriate.

Consultation prompt	Inputs extracted to inform themes
	<ul style="list-style-type: none"> • There is a lack of horizon scanning and pre-emptive planning of new and emerging technologies. Submissions are reactive, from the local sponsor, and are not able to be driven proactively by patient or healthcare needs. • The increasing use of surrogate outcome markers in clinical studies creates a challenge in terms of assessing benefit. This approach is not sustainable where there are comparatively effective, proven lower cost options available. • Currently, there is no mechanism for interested parties, who are not sponsors, to provide feedback into review system, to identify issues and or identify efficiencies. • In addition, there are decisions being made around new medicine PBS listings which require a patient to be hospitalised to commence treatment. This creates a cost for the acute system, but there is no mechanism for the hospitals to indicate their ability to participate, and no forewarning to assist in budgetary procurement.
Current/future barriers to equitable access	<ul style="list-style-type: none"> • Inconsistent access across states and territories may result from variations in decision making due to the heterogeneous capacity and expertise of decision makers, differing processes for assessment of evidence (e.g. NHRA threshold of \$200,000 for high cost-medicines), and pressures to approve medicines for individual patient use. Not all S&Ts have resources to assess novel technologies at a local level for implementation in public hospitals. • Costs for HSTs funded under the NHRA can be a significant burden to small and medium jurisdictions. The opportunity costs for the healthcare system needs to be considered. • Disconnect between regulatory approval and funding approval creates a space for inequity. Where products are approved for marketing but are yet to gain a PBS approval, it creates a postcode lottery of access, where: - those wealthy enough can access new treatments - those who have access to medication access programs can access new treatments - those who live in jurisdictions that support funding can access new treatments. The Council of Australian Therapeutic Advisory Groups do attempt to improve communications between jurisdictional medication governance groups, in an attempt to minimise this inequity. • MSAC has a cost-share mechanism with states and territories. Differences in approaches between MSAC/PBAC can undermine the Government stated principle around the HTA process as being transparent and independent. Under MSAC, it is possible for states and territories to provide input and information into the decision process. This is not available in the PBAC process (outside of consumer comments), and there is an opportunity to alignment. • Lack of transparency in the processes and a risk of key stakeholders not being appropriately consulted during the HTA processes. This can be compounded by a lack of transparency from Drug Sponsors limiting this transparency of information that can, and would, inform decisions of HTA committees due to other factors such as commercial in confidence. • Access to medicines (subject to shortages) via Section 19A (S19A) approvals process allows for timely access, but it does not result in equitable access as patients are required to pay private prices for medicines that they would usually receive subsidised under the PBS. There may be consideration for an acceptable cost threshold guidance for S19A products, as evident by the PBS listing of S19A Tenecteplase at a significant 275% AEMP mark up to the ARTG critical shortage product, though noting it use is in the critical, acute setting. • HTA pathways are optimised for more traditional products and services, however they are often not sufficiently flexible to manage therapies that do not meet the conventional pathway requirements. This can result in some technologies not achieving funding via MSAC/PBAC which places burden on states to provide those technologies, or for them to be bought via the private market and therefore inequity of access. • Access to some HST includes upfront payment for supply by the public hospital/patient with cost-share programs only providing free supply after the time when the patient is expected to have progressed or died and programs which only include limited time bound supply with an

Consultation prompt	Inputs extracted to inform themes
	<p>expectation of further supply to be paid for by the public hospital or patient. This creates “post code” prescribing where one hospital may approve the expenditure to cover these costs whereas another is not able to.</p> <ul style="list-style-type: none"> The PBAC does not explicitly consider medicines used in public hospitals although there have been some PBAC recommendations for medicines which require initiation in hospital. Inpatient use of medicines in public hospitals is generally funded through state and territories, Further, the PBAC does not currently consider the impacts on hospital budgets arising from PBS listing of new treatments either via requirements for commencement in hospital or arising from treatment of side effects requiring admission. There is a significant opportunity to build on the existing models of collaboration and develop a national approach to health technology assessments across the continuum of care so patients can access safe and effective treatment no matter where they are in their clinical journey.
Elements/feature that detract from person-centredness	<ul style="list-style-type: none"> Lack of ability to openly feed into the PBAC process for interested parties who are not sponsors. Person-centred care should not be based on location (of where services are provided) of patients or options of where they receive treatment. The clinical evidence translation to Aboriginal and Torres Strait Islander population and the illnesses that disproportionately affect them is limited. It must be recognised that durability of clinical effectiveness of these newer developed agents will continue to evolve. This further supports the need for robust post marketing surveillance and review as the potential savings realised from ongoing effectiveness of these agents can be confirmed with time.
Perverse incentives	<ul style="list-style-type: none"> The early funding of HSTs for rare diseases is providing a perverse incentive for sponsors to submit applications to funding authorities with relatively immature data. Early access to therapies needs to be balanced with the appropriate evidence required to support the decision, or local evidence generation to inform future decisions. The projected costs for pipeline HSTs will pose a significant challenge to Commonwealth and State Budgets. Government is responsible for the health system’s future financial sustainability, to enable it to continue delivering safe and quality healthcare.
Areas of interest	<ul style="list-style-type: none"> CADTH, ICER and NICE working together, for example to produce a joint position statement on Confidentiality of clinical evidence informing health technology assessment decision making Proactive horizon scanning of new and emerging therapies. For example as undertaken by CADTH in Canada (https://www.cadth.ca/horizon-scan) and the NICE Innovation Observatory in the UK (https://www.io.nihr.ac.uk/). Noting, that it is recognised that medicines commenced by hospital specialists influence prescribing in primary and secondary care, there is also opportunity for significant mutual advantage from greater HTA collaboration between the acute sector, PBAC and MSAC. Another key barrier to access is the required transition of the new health technology into the activity based funding classifications by Independent Health and Aged Care Pricing Authority (IHACPA). While it is acknowledged that this process is likely out of scope for the current review, it should be noted that the transition process is prohibitively slow and complex and delays adoption of new services into standard arrangements. While block funding arrangements are acknowledged as an important interim funding arrangement to determine average costings, these arrangements are administratively challenging and time consuming at a state level.
Other	<ul style="list-style-type: none"> The key concerns with the current process that lead to barriers, inequality and lack of person centeredness is: • Lack of understanding of the process by stakeholders, • Long lead time for approvals and subsequent listings, • Lack of transparency and broad stakeholder engagement • Financial criteria review

Other (n=8)

Consultation prompt	Inputs extracted to inform themes
Elements and features that are working effectively	<ul style="list-style-type: none"> • Terms of reference (TOR) articulate well how the Health Technology Assessment (HTA) Review is positioned simultaneously with other HTA reform processes undertaken • PBAC structure works well for simple single non-complex drug approvals • Value for money • MSAC streamlined approvals process • Project Orbis • Opportunities for stakeholder input • Outcomes for consumers are generally positive
Current or future barriers to earliest possible access	<ul style="list-style-type: none"> • Lack of commercial incentives • Inadequate returns on investment for repurposing • Regulatory barriers for repurposed medicines (fee relief, regulatory support, streamlined submission, exclusivity periods) • Lack of coordinated public funding for integrated and translational research • Need to support more efficient clinical trials • Consider new approaches to monitoring safety and effectiveness of new treatments • Timeliness and processing times • Slow approvals for certain types of therapies, drugs • Lack of approval for combination therapies when more than one pharma company involved • No planning for future drug development • multiple submission for each drug are required and approvals are static and dependent on company sponsors • Inability for organisations/ individuals to request PBS funding outside TGA approved label • Cost minimization is main driver • No opportunity for stakeholder reply • current technologies covered by the HTA are too narrow. • Limited flexibility • current criteria for the assessment process are not fit-for-purpose when it comes to digital health • How to assess when no direct comparative product in market • Cost of assessment for new technologies or innovative products too high • Implementation and uptake challenges and access to customers • implements a one-size-fits-all approach to several very different sub-sectors • The high cost of launching a product in Australia • Clinical value is difficult to demonstrate for antimicrobials • “lowest cost comparator” policy approach to HTA is particularly problematic for novel antimicrobials, • Multiple submissions and reviews – vaccines (av time 1375 days!) • Need greater expert opinion at all stages • Lack of clarity

Consultation prompt	Inputs extracted to inform themes
	<ul style="list-style-type: none"> Reporting needs to be more timely
Current or future barriers to equitable access	<ul style="list-style-type: none"> Precision medicine technologies do not fit neatly into the HTA process Age limits on PBS listed medications in conditions that affect both adult and paediatric patients Lack of paediatric formulations such as liquids, suspensions etc that are evaluated and PBS listed Slow time to reimburse following TGA approval Little opportunity for non-industry entities to apply for approvals Lack of transparency Lack of clarity around how decisions are made no dedicated pathway for “digital health” technologies under the HTA system
Elements and features that detract from person centredness	<ul style="list-style-type: none"> Patient involvement in HTA is limited Patient insights need to be captured equally across skin disease areas and skin cancer, and that patients in priority populations or those with limited health literacy can provide input. All approvals are “disease-based”. A more 'person-centred' approach would be to have drugs approved that are based on the molecular abnormality the patient's cancer harboured. A classic example would be BRAF mutations. Lack of transparency, meetings are held in secrecy with submissions and contributions behind a wall of bureaucracy Lack of community education and opportunity to contribute No weighting to patient contribution. The process is inequitable and favors those with the 'right disease' or access to the 'right advocacy group'.
Perverse incentives/Unintended consequences	<ul style="list-style-type: none"> New pathways are needed to ensure that the value of innovation and clinical effectiveness is recognised and to ensure that treatments and therapies which may not fit neatly into the system are assessed efficiently to avoid any unintended consequences. A major problem relates to combination therapies. If a pharmaceutical company licences two drugs then they are more likely able to provide a more cost-effective combination - compared to when they may have to work with another company. New therapies/regimens are reimbursed, giving patients/doctors more treatment options, some of those that are reimbursed have no material impact on improving patients' outcome and the decision for this approval is not made transparent. For example, Elo-Rd, or VRd for transplant in-eligible patients.
Areas of interest	<ul style="list-style-type: none"> Role of AI in HTA For Digital Health look to overseas (US, Germany and France) for reimbursement models
Other	<ul style="list-style-type: none"> Existing incentives could include extended periods of market exclusivity and tax credits for research and development. Brain/money drain from Australia, when their ideas are eventually approved by FDA – albeit controversially. Medical publications and peer review are letting us down

Appendix 4: Consultation Forum Meeting Notes

All notes are as provided by *the* HTA Review Team at the Department of Health and Aged Care *for consideration as inputs to the themes.*

HTA Review Consultation Forum 1: Meeting 1

24 May 2023, 2:00pm – 4:30pm AEST

Attendees:

Adjunct Professor Deb Picone (Facilitator and RC Chair)
Professor Andrew Wilson (RC Member)
Ms Adriana Platona (RC Member)
Ms Elizabeth de Somer (RC Member)
Professor Andrew Roberts (RC Member)

AusEE Inc.:

Sarah Jane Gray

Genetic Support Network of Victoria (GSNV):

Monica Ferrie
Julie Cini (Spinal Muscular Atrophy Australia Inc.)

Apologies:

Dr Dawn Casey (RC Member)
Ms Ann Single (RC Member)

General submission statements / stakeholder opening comments

GSNV noted the following:

Patient communities are committed to having a responsible and accessible system that is transparent and delivers the objectives of this review, which includes timely and equitable access to therapies.

There are concerns that the current Health Technology Assessment (HTA) process and methodologies are not sufficient for advanced technologies.

Equitable and timely access to therapy involves more than a review of the HTA policy and process; Implementation needs to be considered in order to meet the objectives of the review. The integration of how decisions are implemented is an important factor in achieving equitable and timely access. Unless implementation (data, evidence, alignment between all contributors and people impact) is considered in HTA process, the impact will be minimal.

It would be beneficial if there was more clarity around the recommended pathway the therapy should take prior to submission.

The close relationship with Pharmaceutical Benefits Advisory Committee (PBAC) was imperative and instrumental in terms of implementation and receiving feedback about what was required for a successful application.

Patient education on HTA processes and better informing patients in patient organisations about the benefits of and assisting with real-world evidence data collection would be beneficial. Empowering patient organisation groups to collect real-world evidence would also be beneficial to consider in the review.

It's imperative that the collection of real-world evidence is commenced early. A development from this review could be bringing to light that data collection should be occurring. Having real-world data available would have

enabled quicker access to treatment. The real-world evidence piece is vital in moving forward in accessing and implementation of the HTA.

Strong patient support group leaders are vital in arming the patient/consumer group with the skills, resources and education in order to collect real world evidence data to inform a successful submission. However, there are many patient/consumer organisations which do not have strong patient support group leaders. Equity should be given to these groups to build their capacity by arming them with resources and education. We need to ensure that burden is reduced from patient support groups that do not have the capacity and capabilities for real world evidence data collection. Patient support groups that have more resources and capacity have a greater opportunity to be heard, which may be a reflection of the strength of the patient support group leader (creating further/compounding inequity).

We have collected spinal muscular atrophy (SMA) data for 18 years we find that clinicians would doubt and devalue the data we spoke to. It needs to be made aware that the data collected from patient support groups is valuable and coming from a credible source. This could be part of mutual education, where patient support groups are educated on collecting robust data and assessors are educated that they can trust that data.

The views of the patient community and expectations in terms of the role that horizon scanning plays to ensure the system is ready for when a new technology arrives and the role patient communities play in horizon scanning – are that patients are valuable horizon scanners themselves, as they spend a considerable amount of time researching their disease and looking for efficient channels to get information from.

Having disease specific information that is more readily accessible to patients and efficient means of retrieving the information would be beneficial as this is lacking. These issues are also highlighted in the rare diseases space, where patient outcomes are somewhat determined by the resources and people in the team supporting them.

AusEE Inc. noted the following:

In terms of horizon scanning and tracking different pipeline drugs along the way from Australia and other jurisdictions, we have a lot of experience with this and feel that it's mainly been consumer-led where we are the ones to approach industry to discuss our findings and not industry engaging with us. There is a need for the review to consider changing this dynamic and incorporating improved consumer engagement.

Elements and features of HTA policy and methods that are working effectively

GSNV noted the following:

One of the best features of the HTA policy and methods has been the consumer representative, Jo Watson. The work Jo has performed with the community is a fantastic feature of the process and has assisted with several treatment approvals. Jo was readily accessible to assist with answering consumer questions and providing guidance and clarity on submission outcomes. For example, Jo has assisted with providing information regarding negative recommendations and providing guidance on receiving a positive recommendation.

Another positive feature has been the facilitation and assembling of collaboration with consumers to enable smooth movement through the process.

Further education for the patient organisation group leaders to enable more accessible language and information, and clarity would be an asset moving forward with this process.

The government websites have significantly improved, and this is a positive element.

The addition of the concept of 'value of knowing' has been beneficial, as it does demonstrate that there is some thinking of other measures that need to be included in HTA processes. However, there are issues around clarity of the reference committee's definition of value of knowing as this is somewhat inconsistent with our definition.

Access to assistance has really improved, as there has been better collaboration on submission requirements prior to submission. Sally Wortley has been a terrific resource for this.

AusEEInc. noted the following:

There are positive elements of the government website (e.g public agenda, having the functionality to track medicine status and progress on approval through the Medicine Status Website, public summary documents), although the information is limited and vague at times. It would be beneficial if the information was a bit more comprehensive and used more accessible language/methods of communication.

The electronic survey questions (in the PBAC comments section) are consumer centred. It would be helpful if there was the functionality to specify if the person doing the survey was a clinician, patient group or consumer, for example. It would also be helpful if the questions were more flexible and worded differently so that they would adapt to suit the person who is doing the survey.

Positive outcomes resulting from Australia's HTA policies and methods

GSNV noted the following:

I think success is measured by an easily understood outcome by all parties with clear direction on next steps and implementation after a positive decision.

Elements and features of HTA policy and methods that may act as a current or future barrier to earliest possible access and feasible options or suggestions for improvement

GSNV noted the following:

Whilst we acknowledge that there is complexity with implementation, this is an area that needs to be improved. Newborn SMA screening and SMA gene therapy are examples that highlight implementation issues - we have been advocating for the past 18 months for the states to place SMA on the newborn screening panels, based on the recommendation that was made 2 years ago. Having a process that simultaneously runs alongside the HTA process to look at the implementation risks and barriers of this decision is essential.

Clarity and transparency need to be improved around which pathway and process will be used for assessment of therapies early on for the greater good of the community. For example, an SMA gene therapy went back and forth to different committees before a decision was made as to its assessment pathway.

- Professor Andrew Wilson (RC member) added that there were very high patient care costs involved in CAR T, and that was a big issue in deciding the assessment pathway.

A recommendation would be to enhance and better streamline the process. There is importance in having a pre-submission alignment, that brings all policy holders together at the beginning and looks at identifying the implementation challenges that are going to go alongside this treatment or service and exploring every facet of that implementation. For example, a statement in a submission may indicate that people should have access to a genetic counsellor for a service or treatment. However, access to a genetic counsellor might not be factored into implementation, the cost and the impact so this creates challenges for the patient community who expect that they're going to be able to have access to what was initially indicated.

- Professor Andrew Wilson (RC member) added that the Committee is aware of these issues and concerns raised and that they reflect in part the different model and different way the two programs (Medicare Benefits Schedule (MBS) and Pharmaceutical Benefits Scheme (PBS)) operate.
- Adjunct Professor Deb Picone (Facilitator and RC Chair) confirmed that a pre-submission alignment is possible.
- Ms Elizabeth de Somer (RC member) added that the PBAC is limited by the National Health Act in determining different funding pathways, however it may not need to be and perhaps can be changed.

Although there may be barriers in implementing this, determinants of high and low value care need to be factored into the assessment and decision making and potentially made more flexible so that it can account for patients that are happy to receive either high or low value care depending on their needs and expectations. Are we factoring in costs that are high value because of the experts that are expected to deliver that care when the patient community isn't carrying that expectation that those things would be delivered by that level of health professional? For example, patients may be content with therapy being delivered by a nurse where the assessment may have costed it being delivered by a specialist clinician.

- Professor Andrew Wilson (RC member) added that the Minister announced that there will be a major review of barriers to scope of practice of health professionals as part of the Medicare taskforce.

The community voice needs to be represented and identifying who the consumer representatives are and their roles and responsibilities (e.g whether they represent their own experience or a community) needs to be more transparent/visible and the information easier to locate. If the expectation is that the consumer representatives are representing, then there is a need for them to be more proactive in seeking out and consulting with the community rather than the patients/groups trying to seek them out.

- Adjunct Professor Deb Picone (Facilitator and RC Chair) commented that there will be a discussion around these issues with consumer representative Jo Watson and Consumer Evidence and Engagement Unit (CEEU)lead, Sally Wortley.
- Ms Elizabeth de Somer (RC member) commented that there is a parallel process (separate to the HTA review) that is being co-designed with the patient groups, industry and Jo's group (Consumer Engagement Unit) on the enhanced consumer engagement, which will allow another opportunity to think about what enhanced consumer engagement means and looks like.

AusEEInc. noted the following:

It is important to have sufficient time to incorporate consumer comments.

There is a need for early involvement of patient groups. This might be achieved by having a register for patient groups to be able to register their interest in diseases so that they receive a notification that will assist to inform them of the HTA process and information regarding application submissions and agendas.

Elements and features of HTA policy and methods that are acting as a current or future barrier to equitable access and feasible options / suggestions for improvement

AusEEInc. noted the following:

There is currently not an option for submitting an application that is patient advocacy group led (currently only sponsor-led processes), ie. Small groups miss out on making a submission. For example, there are a number of elemental formulas that are used in Eosinophilic oesophagitis (EoE) treatment, and the access stops from age 18 so that people over 18 years of age are required to pay for it. Due to the small cohort of patients that would fit the category, it's too expensive for the sponsor to put in an application. We feel that there is not an option that allows for that equitable type of access when they're out of that indication criteria. It would be beneficial if patient group and medical advisory board support systems had the capacity to make a request to PBAC for an indication change in these circumstances.

- Professor Andrew Wilson (RC member) clarified that although it is possible for other people to make a submission, they're very complex and a lot of the time, access to the information is required which may only be available to a company to put forward that application. Professor Andrew Wilson also commented that most of the submissions from companies are developed by consultants, which are expensive and may explain why other groups (e.g community groups) couldn't access them.

GSNV noted the following:

It is difficult for patient groups to digest complex information arising from the application outcomes from government committees, including the questions that arise in response to an application. The large volume of information that is provided in the application outcome document can also be overwhelming for patient groups. Timeframes are also unreasonable for providing responses to questions. Looking at how patient groups can be supported in this area is essential in addressing and overcoming these barriers.

Health literacy among communities and cultural challenges has a large impact in the contribution that health professionals or industry can make. A problem for us as a patient group and globally, is that it's the white Caucasian English speaking women who respond to surveys and provide us with information. Obtaining a diversity of understanding is a challenge that is likely impacting the outcomes for the diverse population. This also is an implementation issue – if cultural challenges are not understood at the front end, then understanding cultural

challenges of implementation will be unlikely. Understanding these cultural challenges is essential in understanding the implementation issues.

Ensuring that communication is being delivered in an accessible way is integral to equitable access. That includes communication of the HTA process, the requirements of contributors into the process, and outward communication such as the public summary documents.

Ensuring that regional rural communities are also going to have equitable access to decisions (service and drug decisions) is important, as it is more difficult for them and takes them more time to be able to access. There are higher costs associated with them being able to access certain drugs and services.

Equity is complex but it needs to consider other systemic factors that could impact the barriers that are experienced.

It would be beneficial to see an encouragement of different methods being considered for how drugs are delivered. For example, for many of our disability communities, treatments would be much more easily administered by patches.

Elements and features of HTA policy and methods that may be detracting from person-centeredness and feasible options / suggestions for improvement

GSNV noted the following:

The biggest detractor is the lack of clarity around the definition of 'person-centered'. For example, it may not inherently reflect that the person or people has/have any input into the decision making or into the evidence that has been provided. The role of the person in the 'person centered' process needs to be more clearly defined.

AusEEInc. noted the following:

The HTA process may not be well equipped to deal with cases where one drug can treat multiple conditions and have multiple indications. This is especially seen in allergic diseases. For example, there may be cases where a patient has multiple conditions, all moderate in severity but that could all be treated with the one medication. However, that medication may only be indicated for higher severity of those individual conditions (for example). It would be beneficial if the clinicians could make a judgement on deciding which therapy they could apply for that could treat their comorbidities to minimise the use of multiple therapies.

There are also barriers when making the selection in the application where selecting multiple indications is not possible.

- Professor Andrew Wilson (RC member) added that it is possible for a company to apply for a new indication which could be for people who had, for example, dual asthma and eczema, however it would need to demonstrate what the value proposition was for the drug concerned for reimbursement of the medicine. Professor Andrew Wilson noted that one of the challenges with clinician discretion is that it is discretion and how a clinician might interpret the benefits in value may differ from patient to patient. Professor Andrew Wilson noted that this issue is certainly within consideration.

GSNV asked the following question:

Is there capacity (or does this already happen) to have a cumulative learning library that both the submission data and the committee's review of that data becomes a cumulative learning space so that it can be referred to to drawn on commonalities between similar/equivalent drugs or drugs for the same condition?

- Professor Andrew Wilson (RC member) responded by confirming that this does take place, however it does not appear in a public summary document because it would involve commercial and in confidence information.

Elements and features of HTA policy and methods that are causing or could cause unintended consequence or perverse incentives and feasible / suggestions for improvement either currently or in the future

GSNV noted the following:

One of the unintended consequences in the SMA space has been the paediatric inequity and what that has meant for the patient community. For example, there has been a disparity in access to SMA treatments for adults and children and different forms of SMA. This has resulted in detrimental effects for the community.

An unintended consequence for the community is that they receive amazing and fantastic news however there are people that are never going to be able to access it. As part of that pre-alignment, managing community expectations is at the forefront.

Providing results of clinical trial information demonstrating treatment efficacy earlier to the public and having discussions as part of the process can assist in managing the expectations within the community that certain groups (children/adults/different age) may not be able to access treatment because of lack of evidence.

Other information relevant to the Review

GSNV noted the following:

The mechanisms and tools (survey instruments including patient-reported outcome measures (PROMS), quality of life measures, carer measures) via which the evidence is brought to the communities is great, however there is no impact evidence collected on the basis of that data. There are concerns that whether what is being measured and the evidence that is gathered is most effective in determining whether the service should be part of Medicare or MBS or whether a drug should be made available because the tools seem to be inadequate in chasing down impact. We acknowledge that this is a global problem, however creating better measures and collecting and providing information that is going to be of more value in assessing impact needs to be improved.

It would be beneficial if there was further consideration of treatment of conditions for which there is no treatment and how access can be provided to improve quality of life - are there services available through the Medicare system?

- Adjunct Professor Deb Picone (Facilitator and RC Chair) clarified that the consideration of treatment of conditions for which there is no treatment is out of scope of this review. Adjunct Professor Deb Picone added that whilst it doesn't precisely fit into our terms of reference, it is a major issue for the community and therefore discussed further.
- Professor Andrew Wilson (RC member) added that the issue is with what services would be funded through the MBS (provided by a health professional for a medical reason) versus what might be funded to support people with a condition and their families through the National Disability Insurance Scheme (NDIS) (social support scheme). Professor Andrew Wilson added that there is variability in terms of what people are provided with through these different programs and depends on who they are and how they apply.
- Ms Elizabeth de Somer (RC member) commented that it would be important to capture in the notes about how the PBAC handles those other programs that may end up wanting to look at HTA type models that are out of scope for this review but may need some further consideration by the government in the report.

There are expectations about what we should be seeking to bring to the committees, and it involves preventative healthcare, preventative treatments or preventative services. Rare disease is a high cost and lifelong user of NDIS and if we're not investing in that prevention then the number of people entering into or needing the NDIS will continue to grow. There is a need for committees to take that into account or at least be cognizant of it.

- Adjunct Professor Deb Picone (Facilitator and RC Chair) noted that this is the opportunity cost argument, and it will be considered as well.

AusEEInc noted the following:

Whilst we do acknowledge that the sponsor has the option to bring a patient group with them to meetings, it would be beneficial if patient groups had the ability to express their desire to participate in the HTA process and attend PBAC meetings, as the decision is currently made by the sponsor. This would also assist with providing the

patient community type information that the committee may request at the meeting, which could be a defining factor for approval or not approval.

- Professor Andrew Wilson (RC member) added that having the opportunity for this to occur has been a notion that PBAC has supported, however it is somewhat procedurally difficult to deal with because of the issues with commercial in confidence information. Professor Andrew Wilson added that PBAC did have an intention of running a trial of inviting patient groups to have a presence at PBAC to have an opportunity to comment, however this fell through the cracks with COVID and is back on the agenda to do and something that PBAC is keen to trial again, although it may not occur for every drug.

HTA Review Consultation 1 Forum: Meeting 2

25 May 2023, 01:30pm – 03:30pm AEST

Attendees:

Professor Andrew Wilson (Facilitator and RC Member)
Adjunct Professor Deb Picone (RC Chair)
Ms Ann Single (RC Member)
Professor Andrew Roberts (RC Member)
Ms Elizabeth de Somer (RC Member)

Telix Pharmaceuticals:

Simone Leyden

THEMA Consulting:

Dominic Tilden
Koji Makino

Apologies:

Dr Dawn Casey (RC Member)
Ms Adriana Platona (RC Member)

General submission statements / stakeholder opening comments

Telix Pharmaceuticals noted the following:

Radiopharmaceuticals can assist in resolving a lot of the issues that Australia is facing in the cancer space, however access to these therapies and the current Health Technology Assessment (HTA) framework are not conducive to investment in innovation and equitable access.

The Australian HTA system is in a great position when comparing to international jurisdictions in the neuroendocrine cancer space, however there is still more progress to be made.

The HTA framework from a co-dependent technologies (theranostics, diagnostics and therapy) perspective needs further consideration. There is a need for consideration in terms of the value that is placed on the diagnostics in relation to improving care for patients, with better surveillance, better understanding of the disease and treatment plans. Having better access to molecular targeted diagnostics can place those patients onto active surveillance and take them off highly toxic and expensive drugs. Ensuring there is value placed on some of the diagnostics can lead to better therapies.

THEMA Consulting noted the following:

THEMA thanked the committee for its time and for the opportunity to discuss issues of HTA. THEMA noted that all opinions offered are those of THEMA alone and should not be taken to reflect those of our clients.

Resourcing can be difficult in Australia where there is a considerable amount of HTA conducted. There is a need for revising processes of HTA to better allocate health technology assessment resources.

There is a need for evaluation of the assessment processes to improve HTA efficiency leading to better outcomes. A lack of consistency in HTA processes may be attributable to an inefficiency in the use of HTA resources.

Public Summary Documents (PSDs) are becoming more transparent, and this has been beneficial, however having common economic models for a particular disease and further sharing of resources may be one solution for improving HTA efficiency.

There is a need for reallocation of how HTA is conducted (e.g where it is done and which technologies it is done on) to free up existing HTA resources and move them towards areas where more value can be obtained.

It may be beneficial to consider an alternative approach that can better use HTA resources, which avoids duplication of assessment as there is not a 'one size fits all' for submissions.

Elements and features of HTA policy and methods that are working effectively and positive outcomes resulting from Australia's policies and methods

THEMA Consulting noted the following:

The Predictability of the 4-month cycles is beneficial.

Elements and features of HTA policy and methods that may act as a current or future barrier to earliest possible access and that may be detracting from person-centeredness and feasible options or suggestions for improvement

THEMA Consulting noted the following:

The process of getting new technologies into a predictable system will be challenging. Predictability in the form of consistency in assessment processes and HTA pathways (Pharmaceutical Benefits Advisory Committee (PBAC) or Medical Services Advisory Committee (MSAC)) would be highly beneficial.

There needs to be better efficiency of processes and efficiency overall in terms of demands on HTA capacity where there might be opportunities to reduce the need for HTA. For example, certain therapies may not need the same level of assessment as others e.g treatment for psoriasis where there are multiple drugs available.

Building incremental value into the HTA system would be beneficial. The current HTA system may/can recognise incremental values (e.g medicines that are better; new mechanisms of action and alternative ways of drug administration that is seen to have more value or convenience for the patient (e.g the difference between once every 3 months vs every day)), however the direct benefits are often overlooked and not appreciated. It would be beneficial if the difficulties in demonstrating/accepting incremental value propositions were overcome.

- Professor Andrew Wilson (Facilitator and RC member) noted that it may not be HTA not recognising incremental value (e.g it hasn't been demonstrated to be cost effective), but rather the sponsor finding it easier to accept cost-minimisation. Ms Elizabeth de Somer (RC member) added that whilst HTA can recognise the incremental values, it relies on evidentiary proofs of that which are sometimes limited.

It was noted that there is a lack of clarity and certainty around how the application process allows for this to be included. THEMA noted that maybe the effort required to avoid the cost-minimisation is too high for the likelihood of success.

Incremental value can sometimes be understated or is not transparent enough in an economic model and the benefits of value packaging.

Patient involvement in incremental value evidence in submission information that is provided has been limited for us due to resourcing and difficulties with engaging patients, and therefore we have not been able to spend time with patients. Reading patient blogs to gain patient perspectives has been helpful. However, we agree with Ms Elizabeth de Somer's comments that there would be value in the patient experience/values to be included in

submissions, as incremental values are valued by patients as well. There is however difficulty in translating patient involvement into evidence.

There has been difficulty experienced integrating patient evidence and identifying whether it needs to be included in the submission. This is potentially attributable to the lack of familiarisation and clarity around patient data collection regulations, often leading to a lack of evidence collection in the first instance.

Being explicitly encouraged to include patient evidence (e.g. in its own section of the submission) may create better awareness of obtaining patient feedback and would help support the evidence that is presented to the HTA committees.

Telix Pharmaceuticals noted the following:

Imaging/radioisotope therapies are not required by the Therapeutic Goods Administration (TGA) to be a Good Manufacturing Practice (GMP) product. There are complexities and costs involved in manufacturing these therapies as GMP products. It would be beneficial if the TGA commenced looking at the value of these products and if there was consistent regulation, structure and higher standards placed on these products (particularly if these therapies are going to go through phase III trials), as companies are ensuring these therapies are a GMP product (high complexity and cost of manufacturing) to retain standards as there is movement to larger patient populations (e.g prostate cancer). The value placed on these therapies may assist with streamlining the HTA framework around it and increase competition and innovation in this space and ensure that exploration of the uses of these therapies in other disease spaces is possible.

Once there is an approved and reimbursed GMP product (diagnostic or therapy) listed on MSAC for an indication, there should be in place by the TGA adherence to its use, over non GMP for that indication. This will ensure the highest patient safety standards, and increase industry investment in innovation in Australia.

There should be more clarity and predictability on which HTA pathway is better suited to assess these therapies (e.g PBAC or MSAC) and potentially create a more single stream approach for their assessment.

It is beneficial for academics to be included in all conversations, as they are one of the most important parts, especially with regards to discovery and delivery of radiopharmaceuticals

The patient value, perspective, participation and involvement are important and there needs to be further clarity around the definition of value.

Capturing the patient value within the HTA system outside of the data and how values are measured when comparing incremental values of cost or product are key priorities we would like to see. For example, the route of administration (injection vs oral vs patch) and care setting (nurse vs patient administering) may differ between the generic and marketed/'first in market' medicine and these should be taken into consideration where one administration method may be more beneficial for the patient over the other. Submissions do not often capture patient feedback as the application does not request it.

Engaging patient advocates early at the clinical trial design stage and including their voices and experiences in the submission by including their contribution to real world evidence has previously assisted the science, recruitment and value of the trial.

Mandating/enforcing the integration of patient evidence in submissions may potentially assist stakeholders in improving with the process.

- Professor Andrew Wilson (Facilitator and RC member) commented that whilst there is increasing early involvement of patients' assumptions in economic models in submissions, this information perhaps gets lost within the submission and needs to be more transparent.

The incentive to go through the immense process of a large clinical trial and big data sets for such a small patient population is a potential barrier for global companies. Despite having other mechanisms available (e.g. Life Saving Drugs Program (LSD)), quicker and easier access to medicines would be ideal and achieved by having a stepped process for the small patient population circumstance without having to go through the large clinical trial and big data sets.

Elements and features of HTA policy and methods that are causing or could cause unintended consequence or perverse incentives and suggestions for improvement either currently or in the future

THEMA Consulting noted the following:

The perverse incentive that has been created by the system for 'first to market' requires all the heavy lifting getting a drug reimbursed and Medicare item number. Reallocating the HTA resources to appropriately balance that (easy second entry vs difficult first entry) would be beneficial.

In relation to RC member's (Ms Elizabeth de Somer) comments regarding harnessing the consulting services that are available to provide early application advice for a first submission in an attempt to avoid resubmission – an issue with consulting services is that they will never be superior to the information that is provided by the HTA process from committees, however that process necessitates the resubmission that we are trying to avoid.

The best feedback comes from committee feedback of a first submission. One potential solution may be to improve parallel processing, which may facilitate a successful first submission and reduce the rate of resubmissions, as applicants will receive feedback early on. Clinical evaluations can be occurring concurrently with economic model and cost-effectiveness evaluations or at least encourage the companies to do so.

- Professor Andrew Wilson (Facilitator and RC member) commented that this can occur currently, however companies are hindered by not knowing what the registration status is and may be concerned they are going too far without having that particular element.

There have been cases where if a medicine does not have TGA registration, the HTA submission fails and then doesn't receive a positive evaluation compared to if the clinical evidence was there.

- Professor Andrew Wilson (Facilitator and RC member) clarified that this occurs in the minority of cases where there are delays.

There is the opportunity to receive the best possible feedback from PBAC and improving the process so that this can happen quicker would be beneficial.

In terms of improving early feedback, health economists can add value to the economics sub committee (ESC) hearings by answering questions and resolving potential uncertainties.

- Ms Elizabeth de Somer (RC member) clarified that one of the clauses of the agreement is to initiate direct communication between the submitter and evaluator during the evaluation to resolve issues as they go along. Ms Elizabeth de Somer noted that this is in progress and that there might not be capacity for companies to attend the specific meeting that is proposed to debate issues with evaluators in the interests of time. Ms Elizabeth de Somer added that if it can happen before the evaluation is finished that that would be more appropriate.

Telix Pharmaceuticals noted the following:

In relation to the RC's discussion on the relationship between regulatory and reimbursement authority and their potential overlapping roles and the feasibility of streamlining processes – I think that there could be ways of streamlining regulatory and reimbursement processes or giving TGA additional responsibilities to facilitate a successful first submission and reduce the rate of resubmissions.

- Professor Andrew Wilson (Facilitator and RC member) commented that there is no simple solution for streamlining processes and in countries where this has occurred, the mixing of the regulator/reimbursement role ends up leading to drugs not being registered because of concerns about pay.

Elements and features of HTA policy and methods that are acting as a current or future barrier to equitable access and feasible options/suggestions for improvement

Previously touched on.

Other information relevant to the Review

Telix Pharmaceuticals noted the following:

One of the recommendations to come out of the Senate Inquiry was the Centre for Precision Medicine and Rare Diseases within the Department of Health to potentially evaluate theranostics as one example (that don't necessarily fall within the PBAC and MSAC model). We look forward to seeing what this would look like within the Department of Health and how it would function within the current framework.

HTA Review Consultation 1 Forum: Meeting 3

30 May 2023, 02:30pm – 04:35pm AEST

Attendees:

Ms Ann Single (Facilitator and RC Member)
Professor Andrew Wilson (RC Member)
Ms Elizabeth de Somer (RC Member)
Ms Adriana Platona (RC Member)

Canteen:

Angela Wicks
Joey Lynch

Lymphoma Australia:

Sharon Winton

Consumers Health Forum:

James Ansell
Daniel Weber

Apologies:

Dr Dawn Casey (RC Member)
Professor Andrew Roberts (RC Member)
Adjunct Professor Deb Picone (RC Chair)

General submission statements / stakeholder opening comments

Consumers Health Forum noted the following:

From a consumer's point of view, it is difficult to understand the Health Technology Assessment (HTA) system in terms of what is and isn't covered, such that it is difficult to identify if there is a problem. The perception is that Australia has a good and high-quality health care system, however the affordability and accessibility is very limited over time particularly with the increases in cost of living. It is difficult to identify whether there are any mechanisms within the HTA system which are contributing to these problems and therefore offer possible solutions to those problems.

It is challenging to consult with consumers on the documentation received, which can be attributable to unclear HTA processes. There are a number of layers of abstraction within HTA processes, such that a large amount of context is needed to be given at every step when consulting with consumers. There is a need for improved clarity of HTA processes.

Canteen noted the following:

It has been difficult for patients to decipher and digest the information as it is multi-layered and complex and often difficult for those working within the health policy economic space to digest as well.

Canteen's main reason for responding to the consultation is regarding the policy and methods for the Pharmaceutical Benefits Advisory Committee (PBAC) specifically, as this is where we see the most impact. Advocating for change as the impacts are seen is important to the organisation.

Some of the HTA processes are outdated and haven't kept up with the pace of change in cancer treatments. Canteen is particularly concerned about inequitable access for people with rarer cancers, barriers to early access and person centeredness.

There are issues for young people with equitable access to treatment. The cost-of-living crisis is creating situations where young people in particular are not having the financial security to fall back on to access medications. Identifying treatment options, navigating the healthcare space and not having the access to widespread support services as a young person are important issues.

Lymphoma Australia noted the following:

We are very motivated by the consultation/review and we look towards the objectives of the National Medicines Policy (NMP) – equitable access and everyone having the right to access medicines. Whilst the NMP is not part of this review, its objectives are really important.

Lymphoma Australia is an extensive user of the HTA system/process (PBAC and Medical Services Advisory Committee (MSAC)) because as a consumer organisation, we are involved in seeing the evidence and seeing the benefit to the community for the patients we represent.

The barriers of the federated system create inequitable access. There is a need for creating flexibility in how medicines are presented through the approval process in order to contribute in creating equitable access. We are interested in exploring this in addition to global data, rare diseases and rarer subtypes of cancers.

There is a need for better clarity around understanding the HTA process for personalised medicines, and the different layers that sit within the system so that we have got the right to try right through to evidence based.

It is great to see consumer involvement again and defining ways to ensure that's embedded moving forward is really interesting to us.

Elements and features of HTA policy and methods that are working effectively and positive outcomes resulting from Australia's HTA policies and methods

Lymphoma Australia noted the following:

We have been a large user of the current HTA processes and have seen it evolve over the years to increase the consumer voice. This is a positive step for us, and it is great to see there is the Consumer Engagement Unit to be used to support what we are doing. Whilst finding who to speak to in the Department can be challenging, it has been particularly useful having discussions with them if there are barriers to the approval process where they have assisted in helping us to try to understand what exactly the barrier is. There has been an instance where collaborative working with stakeholders (the Sponsor, the Department, and Health Minister) has ensured faster access and the grandfather clause in the application allow access for patient's that missed out previously. These have all been positive aspects and experiences.

Canteen noted the following:

We don't have a lot of examples to provide as we are not exposed to a lot of the positive news stories.

The HTA system is generally working well for high volume drugs, giving free or low-cost medications to treat cancer, side effects and a myriad of other conditions. Where we see it not working so well is with the rare disease cases. In particular, it is difficult for drugs for rare diseases to make a cost effectiveness test.

Consumers Health Forum noted the following:

As mentioned by Canteen, we also find that the positive news stories are not brought to light when the system is working well.

Elements and features of HTA policy and methods that may act as a current or future barrier to earliest possible access and feasible options or suggestions for improvement

Lymphoma Australia noted the following:

Whilst there is a robust and meaningful HTA system in place, we have doubts around whether it is currently in line with systems globally in terms of new and novel therapies, personalised medicine and clinical trials and medicines coming to market. This area needs to be reviewed.

Prioritising the low hanging fruit may contribute to providing better access to Australian's.

CAR T is an example to demonstrate that the system is not potentially ready for new therapies.

Having a federated health system and funding model doesn't necessarily mean that patients are going to have access. For example, a patient may be eligible for an approved CAR T product in Australia, however the state can ultimately prevent a patient from having CAR T therapy. This is an example of inequity and is a problem for our current HTA.

It is beneficial that all stakeholders can put through a submission, however we need to look at global data to retrieve the evidence in addition to looking at our own local data for regulatory approval to occur.

Our experience has been that there are moving goal posts in the current HTA process. This is highlighted when there are discrepancies with approvals for similar applications. For example, this has occurred with our submissions for the lymphomas versus myeloma where it was unclear why a rejection occurred for our myeloma application when the data has come through as particularly good. The comparators that were accepted for our earlier submissions did not necessarily have the same rules applied as for later submissions. When the myeloma submission was going through, 2 other states made the same submission, however they were informed that the state health would not be allowing it in their states. We are concerned that we have a system where we have the states making the decisions through the regulatory process regardless of what the outcome is. We would like these issues and processes to be reviewed.

We are concerned whether our HTA system has the capacity to deal with continuing approvals for novel therapies (e.g gene therapies). For example, do we have a hospital system that is ready to take patients, and will patients miss out because we don't have capacity? We are also concerned that issues with manufacturing processes may prevent patient access in a particular space of time and this will result in wasting of money.

A further issue with our HTA process is the process of monitoring of outcomes. For example, it was a requirement that CAR T outcomes would be captured, and we would be able to access and review the outcomes, however this has not been possible. This is concerning for patients because we are unable to verify if the CAR T therapy is working well for a large percentage of patients. It's also concerning from a cost- effective situation.

These points we have discussed are examples of processes and inequities and the current barriers to an existing policy we have. We have attempted to define ways in which we can consult better to generate a solution to this problem, and this has involved putting together a working party of different stakeholders. We are keen to meet with government and others to try to resolve these issues.

Canteen noted the following:

As we are not directly involved in the application process as a not-for-profit organisation, we don't have a lot of insight into what specific mechanisms aren't working. However, what we do see are the impacts and the outcomes.

There are some issues with the HTA process that indicate that access is not all that timely. There may be other aspects of the application that are too complex, and applicants are potentially not being incentivised enough – ultimately the pharmaceutical companies will want to know that it's going to be cost effective to go through the HTA process.

Another element acting as a barrier is that the range of factors considered in a submission are perhaps not broad enough, particularly in terms of cost effectiveness, which may act as a disincentive to applicants. Applicants may perceive that PBAC will only consider a narrow range of factors and assume that their application will be rejected so won't bother with submitting.

- Ms Ann Single (Facilitator and RC Member) commented on Lymphoma Australia's submission referring to a submission registry to identify those drugs that are particularly being sought. Ms Ann Single also commented on Canteen's range of factors comment, highlighting that it speaks to the social return on investment indicators that were in their submission.

Regarding Ms Ann Single's question on the social return on investment document we previously provided – we believe that there are a few different ways of incorporating social return on investment into consideration – one option is including it in that economic assessment aspect of it. In terms of avoided costs, avoided mental health costs, avoided social welfare costs etc – these are not that difficult to quantify. Other factors that are not easily quantifiable include hope and having the guidance, love and support of parent. The social return on investment report we provided does come up with ways of quantifying those abstract things using proxies so that there is an option to completely have social return on investments sitting under that economic evaluation. The other option, which might be already available but there is just no incentive to use it most of the time, is under section 5 of the report e.g. 'what are the non -health impacts?'. There is also the option to have a completely different pathway for these rarer high-cost drugs.

It is difficult to measure the impact of a person in a community, voluntary persons within an organisation recruited to help other young people and the impact of hope for example in a traditional sense or dollars and cents outlook, however these factors have impact that is currently not covered under the current system. This feeds back into the rare cancer space where there are access issues to those therapies. From a young person's perspective, every cancer that a young person gets is a rare one because young people generally don't get cancer so they're battling uphill in two ways – young people typically have a large number of these factors that come into play and also battling the original issues with rare cancers.

There are a few examples in Europe where various managed early access schemes have worked particularly well that Australia can be looking at as a good example around overcoming barriers to access.

Consumers Health Forum noted the following:

There is a need for better visibility on the HTA process overall and this will assist in identifying where the problems are stemming from.

We understand that for example, we pay more in Australia for medicines than other jurisdictions and that the process for getting approvals through HTA mechanisms is longer in Australia. However, we don't understand the reasoning for this - is this a problem where applicants are taking longer or they're not completing the forms correctly or are there longer HTA processing times because HTA staff are underfunded and there are not enough public servants to get the process moving along?

It would be beneficial if the HTA process could be faster without compromising the quality.

It is beneficial that post market surveillance for these approved medicines is becoming more of an option that's pursued, however we are not necessarily satisfied that there is a rigorous process by which adverse events or problems can be reported and brought back into the evidence space.

We acknowledge that clinical trial access in Australia is limited, however there is a need to improve this in a safe and quality way, which can be difficult to do.

A significant issue for us is that the focus on the health economics of dollars and cents may potentially strip away a lot of the broader concepts of what health is meant to be about and quality of life factors. For example, a therapy may not be approved because it's the same dollar effectiveness for something that is already approved, although the new therapy might a more convenient way for consumers to take it or interact with it or might be a something that is less of a hassle (take at home vs going to doctor or orally vs injection). These factors are not taken into

account during the decision-making process for listing on the Pharmaceutical Benefits Scheme (PBS) or Medicare Benefits Schedule (MBS) or through the rare cancers/disease's pathway.

It is difficult to understand the basis of the decision making and the causes for the delay.

- Ms Elizabeth de Somer (RC Member) commented that the perception that Australia is paying more for medicines than other jurisdictions is possibly a misunderstanding. Ms Elizabeth de Somer also noted that although the public summary documents are published, they are not particularly digestible.

There is lack of clarity as to how the system works and how this leads to the pricing and the pricing changes. A potential solution to assist consumers may be to create understanding of why the same medicine costs more for an Australian government to buy compared to another jurisdiction. The cost effectiveness likely does generally work for the high-volume therapies, however the benefit of therapies for the rare disease patients is immense in terms of health outcomes and convenience. It is difficult for the community to understand the assessment process and rationale for PBAC's decisions for approving or denying these sorts of applications where the therapies are low volume. Consumers will find it important to know that they'll potentially be paying more out of pocket because of the way the system is being structured with reimbursements (e.g safety nets, PBS allowances).

At the heart of all healthcare should be consumer led and consumer decisions and if a consumer wants to try a therapy or not that is their decision and should have the choice to do so and be accessible in a way that isn't going to financially ruin them. The healthcare system should be set to enable them to enact their choices. We acknowledge that evidence is important for enabling this, however often in these sorts of cases, evidence is limited and having mechanisms in place (e.g clinical trials where people are allowed to try things that aren't certain) so that the system is set up to let them keep trying therapies sequentially.

Lymphoma Australia commented that:

There is a need for there to be more clarity around definitions, the different types of pathways and what we are hoping to achieve so that we are all on the same page. For example, what is the right to try and are we all on the same page?; When we're talking about evidence, are we all on the same page? The right to try is important, but when looking at it from a budget perspective as well as from the personal experience of the patient, we have an obligation to ensure that when someone is potentially in the final stages of their life, that we're not giving them hope where there is no hope. We do have to have solid reasoning to enable patients to keep trying therapies in trials because there are ethical boundaries within that.

- Ms Elizabeth de Somer (RC Member) noted that a key point taken out of this discussion is clarity and understanding and that there is variability in what people understand or know or believe or think is available or is not available. Ms Elizabeth de Somer commented that that is a lesson perhaps for the committee to consider - whatever comes out of the review, then how are we going to educate and inform the most important people in our healthcare system (the users of it) about what we are doing and why it is being done? Ms Elizabeth de Somer noted that sometimes it's not that lack of transparency of the system but rather, the lack of translating difficult topics into digestible and understandable pieces of information.

Elements and features of HTA policy and methods that are acting as a current or future barrier to equitable access and feasible options / suggestions for improvement

Canteen noted the following:

There are inequities that begin in trials. This has been touched on previously – we particularly see problems with inequity with rarer cancers. Often, the approvals are for specific age groups or presentation. We find that if you have a rare cancer, you don't have the same level of access to the drugs that you need compared to someone that has a more common illness or cancer. We are seeing issues with young people being in that in-between age group where they are not qualifying for paediatric or adult trials. There is a real opportunity to improve equitable access through clinical trials.

We have experienced difficulties in providing access to clinical trials for young people, as there are frequently young people in the 18-25 age bracket that may be more suited to clinical trials in the paediatric space, however they may not qualify. There are often paediatric trials using higher doses of certain drugs that are targeting cancer types that a young person may be diagnosed with at a younger age, however they are precluded from accessing

the trial as an adult because they are considered an adult patient and thus are required to move into the adult space. For example, a 19-year-old patient may be lumped into the same category as someone in their 60s, 70s or 80s, even though their cancer types are incredibly different. This then affects the drugs they qualify for; some drugs might be only classified for paediatric use vs adult use. There has been a case where a patient accessing a CAR T trial was treated in the Children's Hospital rather than the Adult's Hospital in the United States because it was perhaps determined that their cancer type suited paediatric protocols more than adult protocols.

- Drawing on the above discussion Ms Ann Single (Facilitator and RC Member) highlighted that they noted in some of Lymphoma Australia submissions that some people in their community can fall into a 'no man's land' when either old drugs are the standard of care but not being reimbursed or treatments that may not be particularly expensive but just aren't being brought to market in Australia.

Lymphoma Australia noted the following:

There are inequities that stem from lack of information. For example, we can assume that the majority of Australian's wouldn't know what HTA process is. The problem therefore lies in the challenge that is communicating what the HTA process is, what it involves, what are the ways in which you can navigate the system if you have a health issue. A white paper release today mentioned that a 1-stop-shop portal for Australian's to be able to access and understand the HTA process could be a good step if it's set up in a way that's consumer friendly. It can include information about compassionate access and give information about what PBAC and MSAC is and guidance around key issues.

Another example of inequities that stem from not having information is that depending on where you live in Australia, that can make a huge difference where there is inequity in access to information based on your specific disease and what treatments are available for it. For example, patients may not have been told about treatments or trials that have been recently approved or trials going through the approval process in other cities or states.

Inequitable access is also evident when there are therapies that are going through the approval process, however it is ultimately the hospital that can still make the decision about whether or not a patient will have access to the approved therapy.

We feel as though Australia is very rigid with our pathways and that this can create inequitable access. For example, there has been an instance where a therapy for a rare and terminal type of colorectal cancer was not brought to Australia despite having approval by the Food and Drug Administration (FDA) and in the United Kingdom (UK), due to the sponsor's decision of not wanting to bring it to Australia because it was actually a cheap medicine. There is therefore a need to be conducting horizon scanning and reviewing what is working and being approved and whether the therapies are coming through a sponsor, patient group or clinical group. Ensuring that we create a pathway for patients to access these treatments that have the evidence (the low hanging fruit), is essential in creating equitable access. The group of patients that I worked with all wanted to see a change in the system in this area and would have been alive today if those drugs were brought to Australia.

In summary, what we would like to see in the equitable area is access to information and treatments regardless of where a patient lives and what disease a patient has. Additionally, we would like to see that there are no external barriers that can be generated based on state or hospital.

There are disjointed equity approaches in Australia. For example, where a therapy has not been approved for subsidy, a private patient may be offered the option to pay for the medication, whereas the public patient may not be offered the option. A system should not be allowing that. The patient should be having the right to make a financial decision and not the hospital or doctor making that decision for them. Relating back to the 'one stop shop' portal that was mentioned previously – this could be used as a tool to provide all the options available to the patient so that when someone is diagnosed with an illness, it's in an easy to understand language with all the treatment options explained in one place rather than having to access information from different websites (e.g TGA, PBS, MSAC, Department of Health etc).

- Professor Andrew Wilson (RC Member) clarified that in some cases there are people in the public system getting offered drugs that people can't get in the private system as well because the hospital will decide to fund it separately. Professor Andrew Wilson commented that they have had several conversations of this type with doctors who find it very challenging raising issue of the costs of drugs, particularly if they have already made some judgements about what they think is the likely benefit of the drug. For example,

if they know there's any small likely increment in benefit then they feel torn between whether they should raise it or not in that circumstance.

Regarding Professor Andrew Wilson's comments above, we would like to add that the flip side to the right to have information is that there is grief the family will live with afterwards if they are told about a particular medication they can't afford. Conversely, if the family finds out about a drug they could have accessed, and the person dies then they will live with that and that they didn't do everything they possibly could. Determining how to make this an equitable situation so that the time and place does not influence treatment.

Canteen noted the following:

Additionally, an example of inequity of information we have seen recently is when a medicine isn't approved for subsidy by the PBS, it is then at the discretion of whether that patient chooses to pay for that out of pocket. However, we also see patients not being given the choice because the doctor does not want to inform them that there's a drug available that can potentially extend their life and improve their quality of life for fear of the patient being unable to afford it.

Following up on what Lymphoma Australia commented on, equity can be viewed in another way - it's empowering every patient in the system to access or at least have the information available. Education and equity of access can be seen as another form of empowerment of the patient which should supposedly be at the core of everything we're doing.

Consumers Health Forum noted the following:

In terms of general accessibility, the costs for medicines are increasing and this is not insignificant. Given the cost of living increases and stagnating wages, the cost for someone to purchase a medicine is not insignificant, particularly if they have chronic conditions before they start becoming eligible for assistance and subsidies. This doesn't take into account the extraneous cost if someone is living in a rural area and the nearest pharmacy is a 100km round trip away. These sorts of things can add up if there are no other mechanisms by which someone can access these therapies beyond them just being available in an affordable way. Affordability is the main concern in terms of that access and equity side of things, especially when chronic conditions can severely impact one's income and one's ability to be able to pay for necessary medications.

- Ms Ann Single (Facilitator and RC Member) commented that there are people who feel guilty because they've got compassionate access and some of their friends didn't even though they've put in a similar application. Ms Ann Single commented that this area is worth having a dialogue about across the community because it is an inequity that goes beyond all of the systems that come together.

Elements and features of HTA policy and methods that may be detracting from person-centeredness and feasible options / suggestions for improvement

- Ms Ann Single (Facilitator and RC Member) clarified the definition of 'person-centeredness' as the system trying to look beyond the patient or person presenting medically to think about the wide life of the patient.

Canteen noted the following:

Person-centeredness is at the core of what we are looking at with the social return on investment report. We feel that the process and assessment need to take in a broader range of factors and be thinking about what are the things that matter to people, communities and societies. These things are considered to some extent, however there is scope to expand that and to really be thinking about what the outcomes that patients are looking for and what are the things that they would rate as most important.

There is a lot of public knowledge of us as an organisation for young patients but also looking after the siblings and offspring of cancer patients and the offspring of cancer patients and now moving into the parent space where we provide support to parents with children and young adults within our age range. We see the impact and we hear the stories about how the parent or the siblings' cancer diagnosis affects them and primarily we're providing psychosocial support, but we also hear about how they can't work or maybe one of them has to drop out of school to look after the sibling because the parent can't access a certain treatment or can't work. This has a flow on effect on Australia more broadly because you're losing everything an individual would have become because of a patient or a sibling needing to drop out of school etc. This is difficult to measure.

Lymphoma Australia noted the following:

Social return on investment and taking values into account is a difficult area in the HTA process, because we are still in a trial-and-error phase with it. From a consumer organisation perspective, we're trying to value add in this area because it is about the person and patient. However, do we do that so that it is in a way that becomes transparent but also is of value to the regulators so that it makes sense to them? How do we actually quantify/qualify benefit/value in a reasonable way? There was a patient that managed to fund raise to have access to a medicine, however there are other patients with exactly the same disease who needed the same medicine and couldn't fund raise to get it. It was difficult for us as the advocacy group to try to ensure that every person who needed that medication was actually going to access it before it became approved.

Consumers Health Forum noted the following:

Scotland or the UK or elsewhere in Europe has a specific department within their HTA equivalent process, whose job it is to find people who would be affected by a potential technology and ask them what they think and how it would benefit them and what the improvements are or have been or could be if they had access to it. This helps to inform the decision of whether it's going to be funded or not. It would be beneficial in Australia to have a clear place for consumers or patients who want to contribute and having a clear pathway where it's actively encouraged and enabled (so it isn't just the sponsor's responsibility to find patients).

- Ms Ann Single (Facilitator and RC Member) added that Scotland's HTA system has similar processes to the unit in Australia. Ms Ann Single noted that Sally who leads our unit in Australia is in contact with those people around the world. Ms Ann Single agreed with Consumers Health Forum's comments adding that Patient Voice Initiative directs people across to the Consumer Evidence and Engagement Unit (CEEU) because it's quite hard for people to find people and that's all part of the challenge we have in this space - having the ability to have people to make sense of the patient evidence and to connect them to it is a really important part of it.
- Professor Andrew Wilson (RC Member) added that the first of those engagements involved a group doing exactly what Consumers Health Forum described – going out for any new innovative drug and checking and doing survey work with the patient groups to collect the information around it. Professor Andrew Wilson commented that whilst Scotland has this process, the NICE model is slightly different because it has a separate patient and consumer council separate to its HTA committee that provides a broader aspect on new technologies. Professor Andrew Wilson commented that the group in the department that Ms Ann Single mentioned is very focused on working with the smaller groups (not larger groups like Lymphoma Australia). Professor Andrew Wilson commented that this feeds into other discussions around medicines which don't have a sponsor and how the groups might be resourced to be sponsors.

Lymphoma Australia noted the following:

In relation to my previous comments raised about low hanging fruit areas where if we brought the evidence, there is the question of how do we actually create a pathway for those submissions to come through, whether it's through clinicians or patients? There is a need for us to be resourced so that we can actually meet the standards that we need to meet in order for PBAC/MSAC to make a fair judgement call. This will be difficult to do but is feasible within this HTA review.

A potential solution may be to look at what the other jurisdictions that have consumer or clinician led applications are doing, however that does not mean it will fit Australia. It would be beneficial if the HTA Review considered having a group that could be working on a consumer/clinician led pathway (with the structure around it) bringing applications to the PBS. It may be useful to set up a consumer/clinician led pathway with other stakeholders in there as well. It would also be beneficial looking at some of the best practices of other jurisdictions and potentially modifying that to a potential Australian version.

Elements and features of HTA policy and methods that are causing or could cause unintended consequence or perverse incentives and feasible / suggestions for improvement either currently or in the future

Ms Ann Single noted that this topic was touched on previously. Canteen asked the Committee a question relating to judgements about value – to what extent is PBAC reliant on the applicant bringing that information?

- Professor Andrew Wilson (RC Member) responded with the following: “We have tried to systematise this a lot more. Companies are still encouraged to bring those sorts of inputs in as part of the process, but we are particularly keen to see stuff coming in independent of the companies because what we find is that there’s a broader perspective that can be canvased in those areas. The decision-making process is a separate process to the HTA process – there’s a whole range of inputs that go into the PBAC decision making and the HTA still remains the most important component. We have inputs from individual submissions which are systematically collected and presented – working with the engagement unit, the 2 consumer reps bring individual submission material together and it’s given to the discussant who then presents that material. It also gets presented separately in the meeting -at the start of the meeting there’s a summary of all the submissions we’ve had from patients and patient groups. We also get information from clinical groups. The department will give advice as well from a policy perspective, which may apply to the decision-making process and those things are all weighed up as part of that decision making. Two most important aspects of that from a patient perspective is 1) an understanding of what people see as the value of medicine – the patient experience information is really important in interpreting quality of life data and understanding what it’s about; 2) what is the expectation of what may come from a medicine and where that sits in relation to what the patient sees as the important aspects of their condition – particularly in things where survival is not the key outcome, having an understanding of what those other things are and understanding those aspects are really important in that value judgement. “
- Ms Elizabeth de Somer (RC Member) highlighted that there is a parallel consideration happening (while the HTA review is happening) to codesign with the patient groups, with the consumer education engagement unit and with industry, an earlier patient and enhanced patient engagement process so that they can front load some of that patient engagement and so that the value assessments can be understood before the evaluation takes place rather than afterwards.

Other information relevant to the Review

Lymphoma Australia noted the following:

Patients could be more involved in this space, however they are not really given opportunity to be part of it.

In terms of cost-effectiveness, it would be beneficial for parties if there was the potential of greater patient engagement in what is captured about medicines already in use for post market review.

In terms of budget in the HTA process, we see that there is sometimes wastage in hospitals after a therapy is approved. Looking at ways in which hospitals can be incentivised to avoid medication wastage would be beneficial and may result in some budget windfalls – the medications that are not used could be better directed to patients that can’t access them.

The agenda in the HTA process is not detailed enough so that when a submission is being made, it is difficult to see how it will add value. I acknowledge that this is a work in progress. Whilst consumer organisations do not need to know everything in confidence and everything that goes into a submission, it is important for transparency that consumer organisations have a better understanding of what is going in so that they can actually support the process more productively as well. This might encourage more clinicians to get on board as well. Clinicians are a big gap in this market in the HTA process.

The public summary documents (PSD’s) are really important tools, particularly after a submission is made and you want to know the outcome and why it was recommended or not recommended. We have not shared a PSD with our community because it often gives patients information that they don’t need to know. The PSD’s could be improved to provide more simple summaries of the outcome and explanations of the recommendation. Sponsors are open to providing this information to us, however there should be another channel for that information.

Consumers Health Forum noted the following:

We question the necessity of needing to submit multiple seemingly very similar applications.

For example, after a sponsor applies to the TGA and receives approval, why do they then need to start a separate process with PBAC for PBS listing? It is unclear why these two systems happen independently and would be beneficial if these processes can occur at the same time with the same people, making the system more efficient. An application for a new technology to be made in Australia for example, could be provided with an indicative summary of how it can be approved and adapted prior to HTA submission, which would reduce duplication and burden of work involved in submitting an application for HTA review. Transparency and clearer information around these processes is needed.

Referring to the previous discussion on why sponsors are not incentivised to apply, we feel that there is a need to understand why that is the case. It would be beneficial to investigate whether some of the disincentives can be removed and possibly taken out of the hands of consumers.

- Ms Ann Single (Facilitator and RC Member) added that while it might be clear to a regulator (e.g safety and efficacy and looking at evidence that supports that) and HTA (how the drug fits in the system and looking at clinical and cost effectiveness with a comparator) that they're asking different questions, it's not clear looking from the outside why the two systems occur independently and whether something can be done there to increase efficiencies.
- Professor Andrew Wilson (RC Member) commented that mapping exercises have been previously conducted looking at whether there was duplication between the two systems, and it was found that there wasn't a lot of duplication between them because they're asking different types of questions. Professor Andrew Wilson further added that for TGA approval, they need to demonstrate that it's safe and meets certain quality standards, and efficacy tests but there's not a requirement for them to look at the comparative effectiveness, which is what the HTA committee focuses on (cost effectiveness reasons). Professor Andrew Wilson added that whilst there are different levels of standards in the two processes, there could be ways to more efficiently utilise those processes.

Appendix 5: Consultation Extracts - Recommendations (summarised by stakeholder group)

Patient or consumer organisation

1. Key Recommendations:
 - Consider a fast-track approval process – particularly for potentially lifesaving treatments (including rare disease therapies), which can then be reviewed in more detail before a final decision is made but does not deny access to patients who are deemed by their clinical team to need treatment access quickly.
 - Consider broadening the pool of evidence in decision making. Currently, our HTA system is limited in the forms of evidence that it considers when making decisions with a strong focus on large Randomised Control Trials. However, the HTA system should also consider other forms of trial data and include patient-reported outcome and real-world data. This could also be paired with a process for improved alignment with approvals from other countries. Melanoma Patients Australia would support new initiatives that provide increased flexibility to the HTA processes to consider a wider pool of evidence to help patients access reimbursed new drugs and technologies faster.
 - Set benchmarking targets for submission to listing times and transparently report back on any reasons for delays. This would make the PBAC and sponsor companies more accountable to the community for any delays in listings.
 - Develop a 'Rapid Response Team' within HTA team that can work quickly with sponsors between the formal Committee Meetings to quickly resolve and expedite issues to improve submission to listing times.
2. Recommendation 1: That the Department of Health and Aged Care (DoHAC) undertake a review of policy initiatives and supporting mechanisms needed to ensure that children with asthma have timely and equitable access to appropriate medicines in Australia.
3. Recommendation 2: That TGA and PBAC develop mechanisms to support their parallel consideration of unintended consequences of scheduling changes on equitable consumer access.
4. Recommendation 3: That DoHAC consider how to reform the current process for identifying medicines to be assessed by PBAC for PBS subsidy to make it more accessible to all stakeholders.
5. Recommendation 4: That the Australian Government review the current processes for when and how medicines contribute to the PBS safety net with the aim of increasing equity in access to medicines.
6. Recommendation 5: That DoHAC consider how to reform HTA policy so that it appropriately accounts for the high cost associated with inhaler devices, which are critical to the administration of inhaled asthma medicines.
7. Recommendation 6: That the Australian Government enable access to spacers and masks under the PBS to support equitable access given their importance in effectively and efficiently administering asthma medicines, particularly to children.
8. Recommendation 7: That DoHAC seek to increase equity in access to asthma preventer medicines by:

- Establishing a communications and education support service for health care professionals and consumers in relation to the quality use of medicines to ensure they are used 'safely, optimally and judiciously, with a focus on informed choice'.
 - Developing mechanisms to enable the PBS to provide higher co-payments for more costly medicines – such as ICS - that prevent chronic conditions from deteriorating and that keep people out of hospital and emergency departments.
 - Making costs of medications visible to prescribers by providing real-time data on the relative costs of therapeutically equivalent medicines and by cost per dose.
9. Recommendation 8: That DoHAC reviews HTA processes for MSAC to ensure they support assessment of all costs associated with delivering spirometry in general practice to result in adequate MBS reimbursement.
 10. Recommendation 9: That DoHAC reviews HTA processes for MSAC to establish how they can better facilitate routine access to critical respiratory diagnostic and monitoring services.
 11. Recommendation 10: That the Australian Government include additional item numbers on the Medicare Benefits Schedule for pulmonary rehabilitation for people with complex chronic respiratory illnesses.
 12. Recommendation 11: That the Australian Government increase consumer engagement in TGA regulatory, PBAC subsidisation and HTA processes by appropriately resourcing consumer engagement strategies and tailoring communications to the range of needs and health literacy of consumers. This will require:
 - Putting consumer needs at the centre of all decision-making processes,
 - Being transparent in relation to all decision-making processes,
 - Inviting timely feedback from consumers to all decision-making processes,
 - Reforming the TGA and PBAC parallel assessment process, and
 - Ensuring and resourcing adequate/meaningful consumer representation on all relevant Committees
 13. Recommendation 12: That DoHAC identify overuse of oral corticosteroids in asthma management as a priority, and reduce it by working with:
 - Sponsors to create an oral corticosteroid rescue pack with 10 x 25mg tablets only (with the current provision of 30 tablets to be indicated only for use by people with severe asthma who require OCS for asthma control)
 - PBAC to reschedule oral corticosteroid use to authority prescription only, and
 - PBAC to review processes determining access to monoclonal antibody therapies to ensure that people with severe asthma are not required to use potentially toxic cumulative doses of oral corticosteroids when biologics would be more a beneficial treatment for them.
 14. Recommendations: The HTA system needs a mechanism for enabling early access to effective treatments, similar to cancer drugs fund while the government and industry negotiate on price. Managed access scheme can be formalised where price is set after real world evidence in Australia has been collected. This would create a faster method for assessment of the evidence and access to treatment, that is unhindered

by the price negotiation process. We also need a system that assesses the diagnostic test, or other co-dependent technology, at the same time, in the same process as the treatment.

15. Multiple options for change:

- Look to the FDA and EMA processes to bring the TGA length in line with these.
- Provide flexibility with the PBAC process in terms of medications being assessed as submitted (i.e., not restricted to meeting dates with unrelated submissions grouped together).
- Consider meetings occurring with sponsor input (to avoid recommendations that are not ideal or feasible, therefore avoiding future correspondence, meetings and confusion for the community).
- Increase flexibility in how clinical evidence can be presented for small patient groups.
- Define clearer roles between the TGA and PBAC, working in alignment to remove duplication / un-necessary complication.
- Include clinical specialists (specific to that field) for both assessment and when the details surrounding implementation of a PBS listing are being discussed.
- Allow negotiations to conclude before the TGA process has finished (although if the TGA process length is more in line with other countries, this would be less of a problem).
- Allow Pipeline agreements, providing faster access for patients, saving time and future resources with less meetings needed.

16. The National Blood Agreement objectives should be expanded to include:

- A commitment to innovation and best-practice care; and
- Recognition that innovation in therapies can contribute to cost-effectiveness. We recommend strongly that Australian HTA committees include specialist clinicians and patients at every step of the evaluation process to deliver specialised expertise to underpin decision-making. This is essential to an efficient evaluation process. Hurdles to MSAC evaluation, for example, identifying appropriate evaluation tools, clinical or quality of life outcomes or benchmarks, could be established and dealt with early on in the HTA process.
- Involving affected patients and specialist clinicians in the HTA process at all stages will trigger appropriate contributions from consumers and consumer organisations and assist with stakeholder confidence in the process.
- More clarity and transparency about the approval process is required, so that the stakeholders, including clinicians, the affected community and industry are clearer about the process and the progress of specific proposals. - All steps in the evaluation process should be identified and timelines published. - This includes the timeframes for the public release of recommendations to avoid delays in informing the clinicians and community.
- Consideration needs to be given to setting clear and accountable timeframes for MSAC and any other required processes to evaluate new therapies.
- Advice from experienced specialist clinicians should be prioritised over generic clinical evidence at MSAC and before treatment products are added or removed from the National Product List, including the realistic time required for the transition to new treatments – to discuss it with their

patients, change protocols and implement new access procedures, e.g. home delivery or delivery through a community pharmacy, and to ensure the protection of cold chain processes.

17. The HTA system needs a mechanism for enabling early access to effective treatments, similar to the cancer drugs fund (UK), so that people do not miss out on access while the government and industry negotiate on price. Managed access scheme can be formalised where price is set after real world evidence in Australia has been collected. This would create a faster method for assessment of the evidence and access to treatment, that is unhindered by the price negotiation process. We also need a system that assesses the diagnostic tests or other co-dependent technology at the same time and through the same process as the treatment. Incentives, systems, and processes to encourage better communication and collaboration between PBAC, MSAC, industry, and government. There is need for further recognition and planning to be put in place for the future role of genomic tests in treating cancer. There are complex regulatory and cost barriers that need to be addressed now so that there are no barriers to accessing these tests as evidence further emerges as to their usefulness in determining treatment pathways.
18. NACCHO believes:
 - that providing more and targeted opportunities for consumers and other stakeholders to be aware of planned discussions around PBS listing/de-listing/re-submissions and to be involved in the conversation as soon as possible is needed.
 - Providing opportunities for increased Aboriginal and Torres Strait Islander involvement e.g., through committees and consultation processes, along with further dedicated and funded support to assist all stakeholders on how to navigate the HTA processes.
 - NACCHO would also be supportive of the development of another funding mechanism, potentially outside of the PBS process, which might specifically support more timely access to new technology or medications to allow for earliest possible access for a specific population group.
 - Providing opportunities for stakeholders, such as service providers and peak bodies, to provide input into price negotiation processes and thereby potentially help identify alternative approaches to supporting earliest possible access for items for potential listing under PBS or MBS is also recommended by NACCHO.
 - We support inclusion of NACCHO in pipeline development and horizon scanning activities, where future technologies that may benefit Aboriginal and Torres Strait Islander people may be supported by stakeholders and government to improve the chance of accessibility.
19. Key Recommendations: Strengthen focus within the HTA processes on rare cancers such as Ocular and Mucosal Melanoma to improve equity of access to treatments for patients. Provide financial incentives to encourage sponsor companies to invest in research and the development of novel medical technologies in Australia to improve outcomes for patients with melanoma.
20. Recommendations: an exploration of international processes and practices that are working effectively and could include:

- clear pathways for regulatory approval and reimbursement of new and emerging advanced technologies, aligned with international processes.
 - stronger orphan drug incentives to bring Australian orphan drug designation in line with other countries, in particular offering advice and assistance to small companies that have not previously interacted with the Australian regulatory system.
21. Recommendations: Incentives need to be in place for companies to apply for regulatory approval and give patients access including:
- clear pathways for regulatory approval and reimbursement of new and emerging advanced technologies, aligned with international processes.
 - stronger orphan drug incentives to bring Australian orphan drug designation in line with other countries, in particular offering advice and assistance to small companies that have not previously interacted with the Australian regulatory system.
 - a program like the FDA's 'Rare Paediatric Disease Priority Review Voucher Program'³ should also be considered. A unified patient registry or network of patient registries is urgently needed for the rarer neurological or neuromuscular diseases and disorders. Could be expanded to include dementia etc Data capture from existing medical records should be integrated into a resource that allows accurate identification and characterisation of the patient population for clinical trial planning. Could be enabled to allow for expanded access to clinical trials.
22. Recommendations: Be more flexible/ shorten the process when it comes to expanding a listing for a small sub-group. Look to comparable data or in vitro data if a clinical trial is limited in these sub-groups. Patient outcomes / need should be better accommodated with a pathway available to negotiate based on specific (non-trial based) patient needs such as for Fiasp and Cayston.
23. LFA recommends the adoption of the German and UK models and places a legislative requirement not exceeding 180 days from approval to prescribing. Once a treatment has received TGA approval, it is considered safe and effective, and could be entered into an early access programme to enable earliest possible access for the people.
24. We would recommend more priority should be given to these patients (rural and remote), as well as incorporating "Equity of access issues" such as age, socioeconomic and geographical status, into its own factor within the PBAC decision-making factor matrix.
25. Key Recommendations:
- Work to continue to develop a new and refreshed consumer-led process which is co-designed by consumers and consumer groups and puts consumers closer to the HTA decision making processes as outlined in the 2022 Strategic Agreement commitment between the Commonwealth and medicines industry to deliver enhanced consumer engagement with respect to applications to list new medicines on the PBS.
 - Provide independently reviewed plain language, transparent summary documents for each listing which are aimed at consumers and consumer organisations. These summary documents should

provide factual information and links to key data sources that are going to be relied upon during the HTA Assessment process.

- Provide meaningful feedback on the consumer comments process and how this has or has not supported the Committees in their decision-making processes. This needs to go beyond a short mention in the PSD and must make mention of how the consumer perspective and lived experience was considered in the decision-making process. Committees need to be honest as to whether consumer feedback has been valuable and explain why.
- Improve transparency and equal opportunities for Consumer Hearings to be used in the HTA processes.
- Develop a register of accredited consumer groups that have been trained and commit to ongoing development to contributing to HTA processes. HTA should consider providing grants to accredited consumer groups to help gather independent consumer input from their community, especially for high impact listings that require significant time and resources.
- Provide training and examples of what good consumer comments look like and provide opportunities to educate and empower consumer groups on how to add value to HTA processes. Co-design with consumer/consumer groups a suite of best practice guidelines on how to add value to the HTA processes through the consumer comments process.

26. Recommendations: a revised process could include enhanced communication with the peak organisations that represent these patient cohorts as this is an underutilised yet efficient and effective communication pathway. Sponsors should be empowered to increase patient involvement across the breadth and depth of the HTA process. This could include working more closely with patient organisations to alert them of the sponsors intention to:

- seek registration of a new medicine with the TGA at the beginning of the process. The information provided should include summary information about the eligible patient population, plain language detail as to the clinical studies, key clinical outcomes and what they mean for patients and any safety considerations.
- submit to PBAC. Including the provision of any additional information not provided at the time of registration with the TGA. We note that this is currently standard practice but is often reliant on relationships rather than being process driven.
- withdraw or somehow change existing listings on the PBS, so that patients can be alerted in a timely way.

27. We recommend consulting with Half the Story – A Guide to Meaningful Consultation with People Living with Dementia, Families and Carers, when considering opportunities to improve consumer engagement.

28. Recommendations: As the general community are reading the PBAC outcomes, the technical information could be written in clearer more accessible language. Separating cost effectiveness from price negotiations would allow the information (recommendations) provided to the community to be far easier to understand (e.g., less about patient numbers and similar details, effectively meaning the community

would be less confused and anxious about the process). An ability to submit personalised trial data for those outside listing criteria to expand the listing.

29. We recommend the International Association for Public Participation (IAP2) be the standard for consumer engagement across the spectrum.
30. Key Recommendations: Improve rules and regulations surrounding compassionate access programs used by sponsor companies during the HTA process in Australia to better protect consumers. Consider a co-funded compassionate access program for patients that need urgent access to a new treatment or technology. This could include a government co-payment for the treatment which would be refunded by the sponsor company if the treatment is not successfully listed within an agreed time period. To improve equity of access, this program should provide incentives to support patients in regional, rural, and remote Australia to gain access to the new technology or treatment.
31. Recommendation: Australia should explore the creation of an early access programme similar to the UK's Cancer Drugs Fund, or managed access models, such as the German model.
32. We recommend considering stronger requirements for data on real world outcomes in older people, ideally characterised by multimorbidity/polypharmacy/frailty, and considering specific, including patient reported, outcomes, as well as global health outcomes, to inform routine reassessment of HTA decisions.
33. We suggest that HTA policy and methods are very clear about what information or data is required and who is responsible for this data collection. In addition, we emphasise the increased use of lived experience and qualitative data, including patient reported outcomes and quality of life data. An example is Project HERCULES in Europe, led by Duchenne UK, which includes the development of patient-reported quality of life measures and considers quantifying the burden of illness. Finally, the associated costs of side-effects of medications or treatments must also be included in adjusted 'real costs / real benefits' of a treatment.
34. Suggestions:
 - Automatic or more streamlined PBS listing approval of equivalent medications which have received s19A approval.
 - Greater consideration of equity needed in the HTA process, including greater PBAC and government flexibility in novel and niche medicines e.g., more Department led/NACCHO-supported PBAC submissions, particularly noting that Department led MSAC submissions occur.
 - Transparency and probity of committees and sub-committees and robust sector consultation are important at all stages of the HTA process.
 - Actively seeking early NACCHO feedback on whether PBAC or MSAC applications may potentially impact Aboriginal and Torres Strait Islander people and may require additional scope and stakeholder engagement.
 - A managed access pathway for preliminary PBS listing where a medication without standard clinical trial effectiveness data could be approved for preliminary listing for Aboriginal and Torres Strait Islander people under PBS, with ongoing PBS listing based upon real world evidence. We are concerned that managed access programs (MAPs) alone are not a sustainable means to supporting equitable access of medications for Aboriginal and Torres Strait Islander people. The

proposed preliminary PBS listing pathway approach builds upon the approach taken by PBAC in relation to listing ipilimumab on the PBS with the condition that the sponsor work with clinicians to provide two-year overall survival data of patients who accessed ipilimumab over 12 months. It is suggested that the preliminary PBS listing pathway consider conditions such as providing patient specific data (such as overall survival data, adverse event data, hospitalisation data), sentinel site data, supply data - where applicable. Specific example: PBS listing of Ozempic, Trulicity and other medications for diabetes for Aboriginal and Torres Strait Islander people under 18s (noting that current use is off label). This pathway could also be informed by recent international guidance available around RWE developed by ISPOR, EMA, UK NICE, Health Canada and International Council of Harmonisation. Importantly, this preliminary PBS listing pathway could support existing health inequities whilst also acknowledging and valuing Aboriginal and Torres Strait Islander cultural practices and perspectives e.g., through consideration of Aboriginal traditional medicines.

- Increase clarity and visibility of PBS medications to be de-listed with de-listing criteria to include consideration of impact on priority population groups and input from those potentially impacted as part of decision-making process, along with additional measures to enable ongoing access if there is a clinical need for specific population groups.
 - Potential involvement of service providers, peak bodies and stakeholders as part of price negotiation (for PBAC and MSAC submissions).
 - Incorporating forcing functions to ensure that Aboriginal and Torres Strait Islander people have preferential access to life saving (or essential ongoing) medications such as prioritising access for Aboriginal and Torres Strait Islander people with reduced PBS restrictions as compared to the general Australian population, similar to the approach taken by New Zealand's Pharmac.
 - As an alternative to a sponsor lead process, a commissioning approach could be applied for medicines with an identified need. For example, nicotine gum and lozenge were de-listed in April 2023 when the only PBS sponsor was unwilling to accept the PBS price, perhaps an open tender process could be run to find a replacement at an acceptable price (similar to PHARMAC).
 - Increase PBAC executive decision-making powers to include consideration of requests with a clear benefit to community.
35. We would suggest more detail on summary information, or the provision of relevant links, about the processes that lead to a submission being made to PBAC.
36. Suggestions: Clearer separation of the price negotiation from the cost-effectiveness question. Elements such as patient numbers from verified sources (such as a patient data registry) should only be discounted in extreme circumstances. Also include things like real world overseas data for patient up-take and continuation rather than local data from other medications.
37. Suggestions:
- Requirement that if PBS listed medication is not available, but same sponsor has medication available with brand price premium, that brand price premium cost be withheld.

- Automatic exception of PBS Aboriginal and Torres Strait Islander list for statutory price reductions.
 - Automatic fee waiver for PBS listing requests which may have a substantive positive impact on access for Aboriginal and Torres Strait Islander people, inclusive of PBS listing for Aboriginal and Torres Strait Islander people.
 - Broader consideration of how Aboriginal and Torres Strait Islander people can access medications and medical aids under other existing (or future) programs with a focus on equity and reducing any existing perverse incentives due to program implementation guidelines.
38. The Department and PBAC should be able to request that the TGA review and extend a product's indication where it may address a significant unmet need. Longer term, consideration should be given for a single approval entity or review to facilitate streamlining of approval processes, and processes for extending indications between the MSAC and PBAC processes.
39. We propose:
- 'Right to Trial' program to support systematic evidence development and evaluation of off label use and re-purposing of medicines.
 - additional reimbursement pathways
 - a harmonised national approach to cellular and genetic therapies.
40. As part of the Review, it is important that new concepts are tested for feasibility to ensure that the outcomes and recommendations from this Review do not inadvertently entrench existing inequities and/or lower the value placed on new technologies. Consideration should be given in assessment processes to the negative impact to patients when decisions are made not to fund technologies or the regulatory hurdles delay patient access. All submissions for funding of new technologies should demonstrate evidence of clinically relevant benefits and patient relevant outcomes, such as improvement in overall survival and quality of life. Regulators should encourage evidence of additional patient relevant outcomes to be submitted such as patient preferences for one treatment over another (through better integration of the patient perspective and experience in submissions), ability to return to work or other meaningful activity, and beneficial effects on family and/or carer. The PBAC can assess evidence from other sources than randomised clinical trials to inform their recommendation. This could be more explicitly set out in the PBAC guidelines as the process is still not consumer focused. Additionally, applicants could be encouraged and supported to submit patient reported outcomes and experiences. Similarly, the Review should identify opportunities for patient outcomes from clinical trial data to be presented to the PBAC in a clinically meaningful way. Other opportunities to improve patient engagement with the HTA process could include a more person-focused online submission process which has standardised questions to capture the elements of their experience patients want to share to support the PBAC in their assessment. Promotion of the consumer portal to clinicians could increase wide professional views to be considered in the assessment process. Resources are needed to address the gap between health technology development and its successful implementation. This includes a coordinated independent national molecular tumour board to discuss complex results from sequencing to guide

optimal therapy, to bridge the gap between health technology development and successful implementation. To offset the cost to the health budget of innovative but expensive medicines and other high-cost products, effective policies could be used to drive the uptake of biosimilar medicines including ensuring patients and their healthcare professionals are educated and incentivised to opt for the more affordable biosimilar medicine, when it is appropriate.

41. With respect to consumer involvement, sponsors could alert patient organisations of:

- their intention to submit at the point of seeking registration of a new medicine with the TGA, including summary information about the eligible patient population, what the clinical studies measured, clinical outcomes and what they mean for patients and any safety considerations
- their intention to submit to PBAC and provide any additional information not provided at the time of registration with the TGA
- their intention to withdraw or somehow change existing listings on the PBS, so that patients and their healthcare teams can be alerted in a timely way
- include a section in their submission to PBAC that describes interactions with the patient population such as focus groups, discussions, surveys, etc about the impact of the new medicine on quality of life, (including the impact on mental health, carers, employment, etc) and share this information with the patient organisation (as it may have a bearing on any submission they will make to PBAC);

42. The Department of Health could:

- Provide brief feedback to patient organisations on the quality and value of their submissions.
- Ensure consumer hearings and stakeholder meetings include the presence of at least two people from the patient community impacted, in addition to any representatives from the patient organisation.
- Compensate or reimburse any involvement of volunteer consumers in HTA processes including sitting fees, travel and accommodation costs and ensure protocols for consumer involvement are implemented; this involvement should be appropriately supported so that people with cognitive impairment are able to meaningfully participate.
- Create pools of funding for those patient organisations with limited resources to enable their development of submissions and their own consumer engagement processes.

43. Terms such as ‘medical intervention’, ‘indication’, ‘comparator’ and ‘public health significance’ are not terms that the majority of the general population know. This form should be simplified, keeping health literacy in mind. For us to be able to collect more valuable information, it would be good if the consultation period was not during the holidays or during the busiest time of year (Christmas). Provide funding for patient groups or consultation with consumers. Weight the feedback of patients/consumer groups with more importance than other aspects of the process. Is there an option for consumers/patient groups to provide their feedback via telephone with a consultant?

Pharmaceutical / medical technology company/ industry association

1. Alignment on HTA parameters prior to PBAC submission lodgement, to help reduce the number of resubmissions, and to resolve funding pathways and implementation plans for complex technologies and areas of high unmet need as early as possible. This could be achieved by replacing the current optional pre-submission meeting process led by the Department of Health and Aged Care (DoHAC) which has limitations around stakeholder attendance, restricted time for consultation and is not a formal part of the HTA process. Instead, a more comprehensive optional pre-submission protocol ratification process could be implemented for complex technologies or areas of high unmet need led by a subset of Committee members, for example the relevant Discussant, PBAC Chair and ESC. It could include elements such as PICO, financial and economic model parameters as well as the opportunity for patient, carer, and clinician input.
2. Real-time exchange of information between Sponsor and Evaluator during submission evaluations, so that the most comprehensive assessment is provided for PBAC consideration. Building on the existing plans for an information exchange pilot, this could be achieved by allowing Evaluators to request further information and clarification from Sponsors in real-time via the Health Products Portal (HPP). There should be provisions for Sponsor hearings at ESC meetings. Requests for specific input during evaluations could also be sought from nominated patients and clinicians to reduce the number of resubmissions and deferrals, particularly for new disease areas or where treatment pathways require clarification.
3. Recalibrate the milestone requirements for parallel processing, so that PBAC recommendations are not delayed due to misalignment with the TGA Delegate's Overview. This could be achieved by re-anchoring PBAC consideration to the end of the TGA evaluation phase at Milestone 5 or working with the TGA to bring the timing of the Delegate's Overview forward by several weeks.
4. Improve the transparency and predictability of PBAC decision making, so that Sponsors can better understand the rationale behind PBAC considerations and incorporate these learnings to improve future submissions. This could be achieved by allowing Sponsors to participate as an observer during the PBAC meeting when their agenda item is discussed. The deliberation of final PBAC recommendations could remain confidential, as would competitor information. Sponsors should also be provided with a copy of Discussant presentations, as well as any communication between the DoHAC, Committee and Evaluators regarding their submission (that is not competitor in confidence). The agenda of all PBAC Executive meetings should be published.
5. Improve post-PBAC pricing governance so that PBS listings are not unnecessarily delayed due to inconsistent or unclear pricing methodology or interpretation. This could be improved by reinstating a pricing methods manual as previously used prior to 2014 to ensure transparency and predictability of negotiated PBS prices. Where there are different interpretations between Sponsors and the DoHAC around the PBAC's pricing recommendations, there should be a pathway to clarify these matters with PBAC in an expedited manner.
6. Ensure value assessments are person-centred, based on epidemiology and clinical practice rather than a risk-mitigation approach. This could be achieved by increasing the willingness to pay to a minimum of GDP

per capita and updating technical methods with greater weighting given to patient preferences and equity considerations. The PBAC Guideline definition of main comparator should be incorporated into the National Health Act definition of 'alternative therapies'. Utilisation estimates must align with the expected PBS population and represent a fair and credible forecast.

7. Facilitate early funded access for high added therapeutic value products while further evidence is collected. This could be done by reforming the current Managed Access Framework, so it is more feasible for Sponsors and delivers for patients. This could be achieved through a range of changes such as greater acceptance of early phase data, or opportunities for a price increase. The current framework is rarely used. Issues from previous agreements include significant risks to the Sponsor such as price reductions with rebates and interest accrued, and no option for a price increase.
8. Align the assessment and funding processes for medical devices with pharmaceuticals, to reducing distortions in the market.
 - For Medical devices: Reference group pricing. The PBS uses reference pricing for generic clusters and for groups of drugs with similar safety and health outcomes that can be used interchangeably (therapeutic groups). Medical devices are assessed on comparators based on the functions of the device, absent the rules imposed by the Pharmaceutical Benefits Advisory Committee (PBAC) on selecting the most suitable comparator. The Government has flagged an intention to regroup the Prescribed List of Medical Devices and Human Tissue Products (the PL) in line with clinical groupings, but this work has been delayed.
 - For Medical devices: Ensuring benefits of competition. The PBS uses two formularies. Formulary One consists of drugs which have only one brand each; Formulary Two consists of drugs which have two or more brands each. When a competitor comes onto the market, prices are reassessed to ensure the consumer benefits from competition. No such mechanism is used for medical devices.
 - For Medical Devices: Considerations on pricing. The PBS has a number of rigorous processes to assess economic value, international pricing comparisons, and post-market reviews. Many of these processes are absent with medical devices, in particular, consideration of international price benchmarks. When setting prices, PBAC has options including reference pricing, cost-plus pricing and other mechanisms to improve public value. Further, the PBS may use risk-sharing arrangements to protect public value, a mechanism unavailable for devices.
 - For Medical Devices: Limitations on usage. PBAC considers the scope for use of the drug beyond any restriction for subsidy, and the extent to which a restriction can be constructed that satisfactorily distinguishes use that is acceptably cost-effective from use that is not cost-effective. In contrast, many items on the PL have been assessed and approved for one purpose but are commonly used for a different purpose. Once an item is on the PL, it must be subsidised by health funds without regard to quality, efficacy, efficiency or safety.
9. Reduce costs of assessment and reimbursement pathways for therapies destined for rare disease patient populations
 - Costs to be substantially reduced or completely waived for orphan-designated drugs;

- Smaller companies with turnover <\$50M annually should be exempt from paying submission fees prior to successful PBS listing. This would encourage smaller companies like ST to submit innovative therapies for consideration that would not ordinarily be commercialised by big pharma.
10. Abbreviate HTA processes for orphan medicines
 - We would ask the review committee to consider that when a therapy is already internationally approved and will only result in Commonwealth expenditure of <\$10M in AU, then these should be subject only to a direct price negotiation. This would cut 'submission churn' and ensure expedited access to patients.
 11. Introduce conditional PBS listing pathways for medicines with encouraging Phase 2 data in areas of high unmet need.
 12. The Department of Health should adopt a protocol for evaluation of blood and blood-related products in-line with that for other pharmaceuticals
 13. The Department of Health should provide specific guidance and documentation to explicitly allow a parallel registration-reimbursement assessment process for blood products, and comparable with other medicines
 14. The National Blood Authority develop fit-for-purpose processes, to ensure expedited access to new blood and blood-related products. This should include:
 - Development and publication of an appraisal calendar with meeting dates, deadlines, opportunities for input (from sponsor, healthcare professionals and consumers) and notification of outcomes
 - Creation of an independent blood product expert appraisal committee with membership appointments based on expertise in the blood sector, and related therapeutic areas
 - Establish comparable implementation timeframes and transparency in outcomes to PBAC
 - Leverage existing HTA procedural architecture to harmonise processes between the NBA, JBC, MSAC and PBAC to allow a single point of contact during HTA evaluation and to support expedited resolution of issues
 - Development and publication of clear KPI's, that are benchmarked to international standards
 15. The aspects of the current HTA system that work effectively could be further enhanced through greater collaboration and communication between sponsors, MSAC and PBAC, and the Secretariats that support them.
 - Explicit recognition that, where a medicine has multiple indications, the value of the medicine in subsequent indications can be higher than earlier indications
 - The ability to have direct interactions with PBAC Secretariat and Department members outside of the formal process is reinstated;
 - The opportunity to engage with the MSAC Chair or committee representative as part of routine MSAC Secretariat engagement; and
 - Increased touchpoints with evaluators to ensure greater clarity and address issues/questions in an ongoing manner (analogous to pre-PASC but within current PBAC framework and timelines).

16. HTA assessments should include a broader consideration of value, including societal, to recognise a holistic interpretation of where the benefits accrue (particularly in the case of therapies for rare diseases and curative treatments vs lifelong chronic disease).
17. HTA assessments should allow more flexibility in the evidence base, and greater acceptance of non-randomised evidence, the role of real-world data and surrogate health outcomes
18. Decision making criteria should include higher thresholds for incremental cost effectiveness in rare or ultra-rare disease and complex manufacturing settings
19. There should be a collaborative approach (government, industry, patients) to developing value-based payment models as care evolves from the chronic care model of managing serious conditions, to single-administration curative treatments
20. The necessity for vaccines to undergo a separate ATAGI clinical assessment in addition to a PBAC assessment should be reviewed.
21. If the ATAGI assessment process is retained, then external evaluators should be selected on the basis of their personal expertise relevant to the specific product and disease area.
22. ATAGI must allow sponsors the opportunity to formally engage with them during the assessment process (adopt similar protocols to the PBAC assessment process).
23. A mechanism for the review of the pricing, similar to that available for PBS medications (i.e. not requiring a new PBAC submission), should be developed and available for vaccines listed on NIP vaccines
24. The requirements for lower ICERs for preventative interventions should be reviewed, as this especially disadvantages vaccines
25. Reflect willingness to invest in medical innovation in HTA policies and processes by moving away from an over-emphasis across governments that funding of new health technologies is a cost to government rather than an investment in a social good that delivers benefits beyond the individual to the healthcare system, society, and the economy.
26. Align HTA policies and processes with:
 - The aims of the National Medicines Policy
 - Shared objectives of the Strategic Agreement
 - Recommendations from the House of Representatives Inquiry into Access to Medicines
27. Improve HTA processes:
 - Adopt a horizon scanning process which is well integrated with HTA evaluation and investment processes
 - Provide earlier opportunities for engagement and a shared approach to problem solving to reduce submission churn. Support this by transitioning away from the current iterative cycle of rejection and resubmission to a rolling evaluation process
 - Ensure HTA evaluation processes are efficient and fit for purpose for the technologies being assessed. This includes removing duplication in current processes related to the evaluation of vaccines and therapies for rare disease
 - Address the equity implications and perverse incentives created by the current processes

- RSAs should adopt structures which truly share risk between the government and sponsors and there should be processes to facilitate timely renegotiation of these agreements if required
- Better infrastructure to undertake generation of real-world evidence in Australia is also required to support the implementation of these initiatives to secure earlier access to new medicines and vaccines.

28. Improve HTA methods

- Adopting a more progressive approach to managing uncertainty and sharing risks
- Removing the lowest cost comparator policy
- Co-developing agreed criteria for situations where it is appropriate for second order effects to be included in the base case economic analysis
- Basing the level of reimbursement on the most likely/plausible central estimate of benefits and costs
- Ensuring ICERs used in HTA more appropriately reflect an underlying policy intent towards fast and equitable access to innovative medicines and vaccines
- The base case discount rate should be reduced to be in line with comparable markets and the time horizon used in the economic evaluation should reflect the nature of the disease and intervention under consideration.
- Introducing KPIs which track time to access and the performance of HTA policies and processes
- Commercial in confidence
- Establishing an Innovative medicines fund/accelerated access model
- Creating a mutually acceptable framework for Conditional listings
- Establishing Worksharing agreements with comparable HTA markets
- Adopting a high-level consensus framework on the types of research questions relevant to HTA that can be addressed by RWE to promote acceptance and consistency of the role of RWE in HTA

29. Prior to recommending a MAP as part of a PBAC recommendation, a feasibility assessment of data collection and analyses required to address outstanding HTA questions should be conducted with the details of the MAP finalized and agreed between the parties. The agreement should include sufficient detail on data sources and data analysis methods, particularly for the use of real-world evidence and clear boundaries for meeting/not meeting MAP criteria. Evaluation of the available evidence should be conducted in a manner specific to the MAP research question at hand applying the 'most-reasonable' interpretation of data collected within a real-world setting, rather than defaulting to the most-conservative or applying strict HTA methodology.

30. Regulatory incentives for rare disease medicines have been instrumental in encouraging the development and availability of treatments for rare diseases, which otherwise may not have been developed. Use adaptable pathways which allow for patient and clinical engagement and flexibilities in methods of value assessment.

31. A balanced set of perspectives - all relevant stakeholders, including patients, carers and medical experts should be able to participate in a clear and transparent process that represents a meaningful contribution

to HTA considerations. This should include Medical experts in the specific rare disease under review should be involved.

32. Regroup the Prescribed List of Medical Devices and Human Tissue Products (the PL) in line with clinical groupings.
33. Reduce submission fees for PBAC submissions for orphan-designated drugs.
34. Provide the ability to address questions from the evaluators during the evaluation process in relation to clinical data or analyses presented to avoid misinterpretation since once documented, these can be difficult to alter and, in some cases, may lead to submission deferrals or rejection. 2. Providing for a flexible and responsive HTA process that can accommodate new and emerging data and evidence about medicines. This may include the ability to supplement reimbursement dossiers with new data as it becomes available to ensure PBAC has all available data to assist in its decision making.
35. Allow for economic models to be adapted over time as new evidence becomes available, which may require the assumptions and data inputs originally applied in the model to be updated.
36. Having the opportunity, as is the case in other countries, for face-to-face discussions with the Department of Health and Ageing (and/or PBAC) to finalize the financial estimates related to PBS listing of the medicine after a positive recommendation is provided by PBAC. This would avoid the need for resubmissions to PBAC with subsequent delays to patient access.
37. Actively seeking targeted input from people with lived experience and their caregivers rather than relying on people to submit comments via a portal may improve the quality of information used for decision making.
38. There are multiple solutions that should be considered in the HTA reform regarding ICER thresholds. These include (but not limited to):
 - Agreement from the government to a principle that the PBAC should be willing to accept higher ICERs.
 - Agreement for ICERs to be reviewed on an annual basis to keep in line with the current economic environment.
 - The PBAC (or equivalent) should make its recommendation based on cost effectiveness without regard to budget impact. The expansion of the Committee's decision-making framework to include considerations related to budget impact has compromised the Committee's ability to focus on making recommendations which aim to optimise the health outcomes for Australians. Budgets and decisions on investment should sit with the Government.
 - The PBAC (or equivalent) should collate and publish annually aggregated information about the ICER ranges accepted for listed medicines by broad therapeutic groups (for example, oncology, non-oncology, rare diseases) to allow tracking of the proportion of medications that have been accepted with ICERs in each ICER range.
 - To ensure transparency and consistency within PBAC (or equivalent) decision making, the PBAC minutes should explain the Committee's rationale for each product's accepted ICER (for example, why \$50K/QALY rather than \$70K/QALY and why they thought the ICER was acceptable in each instance). This information should be redacted in the Public Summary Documents.

- Provide greater structure and transparency in how contextual factors such as severity, rarity and equity are incorporated into funding recommendations (including in how the case specific maximum ICER was determined).
39. For all therapies:
- Greater use of pay for performance agreements and the Managed Access Program to allow for collection of Real World Evidence.
 - Removal of off-label comparators.
 - Explicit price premiums and more lenient evaluation criteria for niche populations, such as paediatric indications.
 - Clear pricing timeframes within which the Department will respond.
 - Accelerated Access (as detailed by Medicines Australia).
 - A dispute resolution mechanism for positive recommendations where there is an issue that needs to be resolved and for pricing matters.
40. PBAC should consider comparative safety and efficacy when selecting price comparators and cluster and tier therapies within therapeutic reference groups for pricing purposes.
41. More transparent price premiums for new entrants that demonstrate clinical benefit a subset of patients.
42. Allow for direct application to the Life Saving Drugs Program: no double process with the PBAC.
43. Create horizon scanning evaluations: similar to CADTH- which also incorporates whether the medicine is available for that indication in Australia through local clinical trials, the Special Access Scheme or compassionate access. This analysis will then help identify treatment gaps and provide a better understanding of the limits of compassionate access for Australians.
44. Compassionate access: companies should be reimbursed/or given “credit” towards rebates for compassionate access once reimbursement is obtained as an incentive for broader compassionate access programs or Australia should adopt a reimbursed Early Access Scheme similar to the United Kingdom.
45. There could be a triaging system which allows a price benchmark – perhaps a percentage of the German price - for new chemical entities or therapies in disease areas with high unmet clinical need (similar to the Innovative and Licensing Access Pathway in the United Kingdom) to allow for immediate access while permanent pricing and post-market requirements are negotiated over a 6–12-month period.
46. We also recommend that the HTA Review Committee consider the approaches taken in the evaluation of rare disease therapies in the United Kingdom (England and Scotland), Japan and France.
47. This input and support could be noted as a gateway by MSAC in the form of a letter of support from Australian Pathology, as the peak national body representing private pathology in Australia whereby Australian Pathology members provide input into the application to assure its successful support by MSAC. Without the input of the pathology sector who deliver these tests, the MSAC application is often unworkable, leaving patients with continuing high out of pocket costs for private test fees. This requirement should occur for all HTA assessed pathology companion tests.
48. It is essential pathology providers are consulted as part of HTA assessment processes to ensure companion testing and other new tests (such as genomics) are in line with industry practice and take into consideration the specialist skills and knowledge of the pathologists and medical scientists. It is also

critically important that associated MBS fees and rebates are in line with the cost realities. Through early engagement with the Pathology sector this will assist in expediting the assessment of new tests and enabling Australians to access new treatments, including pharmaceuticals.

49. The HTA Review should ensure that we meet the vision of the NMP, as informed by The New Frontier Report. This will require a commitment to the principle that investing in medicines secures valuable health, social and economic benefits for all Australians, and that this should be reflected in HTA policy and methods.
50. Bold and pragmatic reform should be undertaken to remove the patient access gap so that patients can access medicines as soon as possible after TGA registration. This can be achieved by embracing the concepts outlined in the HTA Review terms of reference – faster, more equitable access, patient-centricity and alignment with international best practice. There are numerous options for reform, which would address issues in three key areas:
 - Commit to delivering faster access for patients through policy and process reform, by:
 - Ensuring all assessment and recommendation processes are aligned to allow for reimbursement from TGA registration.
 - Introducing interim funding for certain medicines.
51. Reform the HTA system to better enable first time success through the PBAC process, by:
 - Frontloading the system through earlier engagement, including patient involvement.
 - Streamlining the interactions of the HTA Committees.
 - Introducing an independent price negotiation process to expedite access for certain medicines, to be mutually agreed.
 - Expanding the current independent review mechanism, or considering an independent appeals process.
 - Ensuring there are agreed, transparent metrics for HTA processes to enable faster access.
52. In addition to the above recommendations, ensure Australia is a first launch country, by:
 - Establishing innovation incentives.
 - Exploring co-developed international work sharing.
53. While the PBAC should retain flexibility in the application of ICER thresholds, willingness to invest in medicines warrants higher ICER ranges, which the PBAC should be willing to recommend. The ICER ranges should then be tracked and published by the Government across broad disease areas and clinical settings.
54. Defining Time to Access: There should be agreement from all parties that time to patient access for medicines means the time from when an innovative medical technology (including medicines, biotherapeutics and vaccines) is registered with the TGA to when it becomes available to patients on the PBS.
55. The Department of Health and Aged Care should introduce integrated and agreed data metrics and a comprehensive system for measuring the time to patient access, and report publicly in line with the National Medicines Policy.

56. Broaden the HTA valuation from a direct health sector patient perspective to include a health and welfare patient and carer perspective, including those with a direct (and indirect) impact on the Australian Government budget.
57. Develop agreed criteria for situations where second-order effects on patients and their caregivers, such as social welfare and carer impacts, should be included in the HTA assessment process, including workable methodologies for the transparent inclusion of second-order effects or patient benefits, in a way that supports equity of access.
58. Lower Australia's discount rate in line with international best practice to recognise the value of preventative treatments and cures and speed up access to them.
59. Develop an agreed framework to ensure that health technology assessments aim to identify the most likely or most plausible central estimate of health and outcomes to form the base-case analysis, based on validated evidence. This should be stated explicitly in policy and methods guidelines.
60. For the purpose of demonstrating clinical superiority and cost effectiveness when head-to-head trials are not available, adopt the methodology accepted in other HTA markets (e.g., NICE, CADTH).
61. The comparator should be the therapy(ies) most likely to be replaced in clinical practice by the new intervention, aligned with other HTA bodies and good HTA practice. This is consistent with the earlier interpretation of the National Health Act (pre-2015). Where there are multiple comparators, the economic assessment should calculate a weighted average price for the new therapy based on the proportion of use that it replaces for each of the comparator therapies.
62. Explore ways to overcome issues facing medicines where the clinically appropriate comparator has been commoditised.
63. Adopt a high level, principles-based framework for accepting and assessing RWE. Develop standards for the utilisation of RWE for post-marketing monitoring in the reimbursement context. This would require enhanced system infrastructure to centralise linked health data and provide appropriate access to stakeholders, including industry.
64. Create a framework for predictable, consistent and transparent incorporation of contextual factors, such as patient and consumer input, severity of disease, equity, confidence in the evidence, assumptions and other relevant factors into HTA decision making.
65. Co-develop and implement a Horizon Scanning Roadmap, detailing the steps all stakeholders must take to implement nationally coordinated horizon scanning, to deliver on the commitment in clause 6.2 in the Strategic Agreement.
66. Seek guidance from the ACCC on competition law to enable discussion between multiple Sponsors at time of submission and PBS listing.
67. Establish a single HTA assessment body for cell and gene therapies to remove current inconsistencies and complexities to streamline the pathway for patient access.
68. Establish a single federal funding source for the product costs of cell and gene therapies, similar to PBS funding of medicines.

69. Streamline and, where appropriate, standardise the clinical delivery of cell and gene therapies to ensure equitable patient access and improved quality of life for patients and autonomy for clinicians to best meet the needs of patients under their care.
70. Identify and track HTA applications for orphan drugs through reimbursement pathways so that rare disease specific issues can be identified and addressed.
71. Coordinate HTA applications for rare disease therapies through a single-entry point within the DoHAC.
72. Recognise in HTA guidelines that, for rare diseases, observational data is the best evidence available for decision making.
73. Implement a direct and streamlined path to funding via the Life Saving Drugs Program (LSDP).
74. Formulate a robust and formal framework for earlier and more meaningful consumer engagement across the lifecycle of a medicine, to deliver on clause 6.3 of the Strategic Agreement.
75. Provide greater transparency on the utilisation of consumer evidence and how it informs decisions made by HTA agencies.
76. Commit to delivering faster access for patients through policy and process reform, by
 - Ensuring all assessment and recommendation processes are aligned to allow for reimbursement from TGA registration.
 - Introducing interim funding for certain medicines.
77. Reform the HTA system to better enable first time success through the PBAC process by:
 - Frontloading the system through earlier engagement, including patient involvement.
 - Streamlining the interactions of the HTA Committees.
 - Introducing an independent price negotiation process to expedite access for certain medicines, to be mutually agreed.
 - Expanding the current independent review mechanism or considering an independent appeals process.
 - Ensuring there are agreed, transparent metrics for HTA processes to enable faster access.
78. In addition to the above recommendations, ensure Australia is a first launch country, by:
 - Establishing innovation incentives.
 - Exploring co-developed international work sharing.
79. Determinants of value in HTA assessment
 - 1. Broaden the current HTA perspective, which is limited to direct health sector and patient impacts, to incorporate a broader Health and Welfare perspective.
 - 2. Clarify the remit for the PBAC so that the most plausible central estimate of health and economic outcomes are utilised for the base case analysis, therefore enabling more first time PBAC recommendations.
 - 3. Adopt the methodology for indirect comparisons accepted in other HTA markets (NICE, CADTH) for the purpose of demonstrating clinical superiority and cost-effectiveness.
 - 4. Reduce the base-case discount rate for PBAC evaluations to 1.5% in line with comparable best practice HTA countries.
80. Fiscal constraints undermining funding decisions

- 5. The Minister for Health and Aged Care provides clear direction to the PBAC that PBS budget impact considerations remain a decision for the Commonwealth Government, and should not inform, nor influence, PBAC recommendations to the Minister.
81. Limited mechanisms for accelerated access and interim funding
- 6. There is the development of a co-created interim funding mechanism.
82. Lack of transparency and national coordination of early horizon scanning of new and emerging medicines
- 7. There is the creation of an enduring national structure for the planning and coordination of disruptive medicines.
83. Differences in HTA pathways for cell and gene therapies
- 8. In agreement with the The New Frontier Report, the Australian Government establish a Centre for Precision Medicine and Rare Diseases within the Department of Health and Aged Care (DOHAC), to ensure that the capacity of the DOHAC is enhanced to provide Australians with timely access to new medicines and novel medical technologies. The Centre should:
 - a. Provide advice to governments on the establishment of a dedicated HTA pathway for cell and gene technologies,
 - b. Outline a simplified HTA process for cell and gene therapies, and
 - c. Review HTA methodology applied to cell and gene therapies, considering international best practice.
84. Lack of sustainable funding for co-dependent technologies with international workflow component
- 9. To address the barriers to co-dependent technologies with distributed models of care, the HTA Review Committee should review current legislative barriers such as the Health Insurance Act 1973.
 - 10. There is the development of a blueprint for the implementation of genomics in Australia to accelerate equitable and affordable access to genomic testing (this could be a function of the Centre for Precision Medicine and Rare Diseases).
 - 11. Review public hospital funding of genomic tests to remove barriers to the use of the tests within the hospital.
85. Evidence for tumour agnostic therapies
- 12. For tumour agnostic therapies, increased acceptability of the best available evidence, or the introduction of interim access mechanisms that enable evidence generation for key uncertainties be considered.
 - 13. That there is greater consideration of excluding testing costs in HTA, notably when the testing technology is anticipated to be embedded into the health infrastructure in the near term, or when access to testing is covered through other means (ie. patient self-pay or clinical trial).
86. Lack of assessment and funding pathways for integrated digital health technologies (DHTs)
- 14. That there is the development of a fit for purpose funding framework for DHTs to enable future patient access to the benefits of these technologies.
87. Limited consumer input into HTA deliberations

- 15. Co-design a new process to elevate the patient and consumer voices in access to medicine, also known as the Enhanced Consumer Engagement Process, as agreed in Strategic Agreement (clause 6.3).
88. Comparators and comparator selection
- 16. The PBAC utilises as the comparator the therapy(ies) most likely to be replaced in clinical practice by the new intervention, aligned with other HTA bodies and good HTA practice. This is consistent with the earlier interpretation of the National Health Act (pre-2015).
 - 17. Where there are multiple comparators, the economic assessment should calculate a weighted average price of new therapy based on the proportion of use that it replaces each of the comparator therapies.
89. That an Enhanced Consumer Engagement Process be co-designed with patients, as agreed to in the Strategic Agreement, which includes an opportunity to participate at PBAC meetings as part of discussions about clinical evidence, to elevate the patient voice in decision making.
- (1) That HTA bodies provide patients with summaries about how consumer inputs are considered as part of decision-making processes;
 - (2) HTA bodies better incorporate patient and carer input by having reference to a broader subset of utility values such as patient preference, patient reported outcome measures, and discrete choice methodology.
90. That HTA decision making incorporate direct patient and carer economic benefits when assessing the cost-effectiveness and budget impact of new interventions and develop methodologies to incorporate broader second order effects into decision making.
91. That HTA bodies ensure that the comparator used in assessing medicines and technologies be the therapy most likely to be replaced in clinical practice by the new intervention.
92. Managed access pathways and conditional listing processes are more broadly and appropriately utilised to ensure equity of access to medicines where there are high levels of data or financial uncertainty for rare diseases that have a high unmet clinical need.
93. The Australian Government should measure the performance of the PBS by systematically collecting, collating and publishing data on Australians' time to access for new TGA approved medicines from date of ARTG entry to PBS listing.
94. To ensure whole of government consistency on investment decisions, the PBAC's willingness-to-pay threshold should reflect the Australian Governments' own advice on the value of life per the recommendation by the Office of Best Practice Regulation (\$227,000 per statistical life year).
95. With the view of reducing the existing patient access gap in Australia, we recommend the HTA Review Committee note the reimbursement pathways and time to access in comparable countries, including Germany and recent reforms in France, both of which demonstrate the possibility of rapid access whilst maintaining a sustainable and fiscally responsible budget.
96. The HTA Review Committee should note the significant regulatory reforms introduced following the 2015-2016 independent expert review into the TGA, including those designed to expedite access to innovative

therapies that Australians need; but given effective access can only be provided by the PBS listing process, it is imperative that this too is reformed.

97. The HTA Review should provide options to fund those medicines approved by the TGA accelerated access pathways noting the accompanying uncertainty around early evidence packages; options could include interim funding outside of existing PBS financing arrangements prior to full transition to PBS listing at a later date.
98. In addition to considering the opportunity cost of adopting a new health technology, the PBAC should also calculate and then publish the opportunity cost of not adopting – or delaying the adoption of – a health technology, specifically in terms of the cost borne by patients and society as a whole.
99. The HTA review committee should consider an independent arbitration mechanism to progress listings for medicines that are recommended by the PBAC where agreement between the sponsor and the PBAC cannot be met.
100. In select circumstances, a formal pre-submission process focused on establishing the framework for the clinical and economic evaluation should be made available.

Research & Consultancy

1. Recommendation: Adopt an alternative pathway for determining PICO as part of PBAC submissions (akin to the current MSAC application process; it effectively determines the appropriate PICO for the medical service/technology).
 - a. or at least a binding decision between the Department and the applicant should be established prior to commencement of the assessment to determine the appropriate population and comparator, and mitigate conflict between the assessment and the Committees at the decision making stage
2. Recommendation: Provide option for Sponsors for a "stop clock" during the response period or to preselect different response times (during evaluation to allow for longer response time or to submit additional evidence).
3. Recommendation: There is a need to setup some sort of consumer/patient reference group that provides ongoing advice to Australia's Health Technology Assessment system. A mechanism is also required to enable focus groups, allowing engagement with the community on at least an annual basis. This could be undertaken broadly and/or with specific consumer interest groups, as required.
4. Recommendation: The number of patient / consumer representatives on HTA committees needs to be increased to at least 3 representatives per committee.
5. The New Frontier report has several recommendations not already mentioned that we support:
 - a. Recommendation 1: to establish a Centre for Precision Medicine and Rare Diseases to enhance timely access to novel drugs and technologies, and to provide advice on HTA pathways for new technologies.
 - b. Recommendation 3: to establish an Office of Clinical Evaluation so that HTA is based on the most up to date global health practices.
 - c. Recommendation 5: to increase the number of health economist in Australia.

6. New Frontier Recommendation 2 is to simplify the process and establish a national genomic testing program for equitable access, which we support
7. The HTA process should consider the introduction of a standard step for review by an Aboriginal and Torres Strait Islander Advisory Group for HTA; identification by Government of communities with unmet need for a specific intervention and facilitate processes for expedited application to provide access if inequity is identified.
8. New Frontier Recommendation 28 is to integrate the patient voice upfront in HTA and to consider making patient evidence compulsory in some circumstances.
9. New Frontier Recommendation 6 is to increase education about the regulation and reimbursement system.
10. Recommendation 1: Embedding horizon scanning into the HTA process.
11. Develop guidance on real world evidence integration.
12. Recommendation 3: Federal investment into a coordinated national program of linking health data for the purpose of improved oversight over current patterns of care and more accurate modelling.
13. Recommendation 4: HTA methods should align more closely with the specific value provided by clinical genomics.
14. Recommendation 1: Use disease reference models for economic analyses that are informed by registry and health administrative data (i.e., RWE) to account the impact of novel therapies beyond a single stage of treatment.
15. Recommendation 2: Develop specific guidelines that explicitly consider equity implications in cost-effectiveness and budget impact analyses.
16. Recommendation 1: Consumer involvement in all PBAC/MSAC committees (i.e., there are no consumers in PBAC ESC).
17. Recommendation 2: Establish policy to collect real-world evidence directly from patients through patient-reported outcomes, registries, and patient advisory groups.
18. Recommendation 1: Enhancing flexibility and contextual evaluation of model types in HTA guidelines for cost-effectiveness analysis.
19. Recommendation 2: Comprehensive value assessment. Develop and establish HTA guidelines that include a comprehensive assessment of both short-term and long-term value, as well as consider broader societal impacts and long-term cost savings. This involves integration of RWE on effectiveness, patient-reported outcomes and cost-effectiveness over extended timeframes.
20. 1. Develop a national strategy that is informed by disease burden and unmet need, and is integrated with strategies for funding innovative medicines, establishing local clinical trials and investing in advanced manufacturing in Australia. Importantly, this should not neglect rare diseases and unmet need.
21. Harmonise HTA policy and approaches across relevant funders in Australia – PBAC, MSAC and state governments
22. Improve signalling/communication with researchers/product developers about the nature and standard of evidence required

23. Identify areas of alignment on priority areas and evidence requirements with Canadian and UK HTA agencies, via work sharing arrangements
24. Monitor clinical development programs through horizon scanning and proactively engage with companies on evidence requirements
25. Remove double cost recovery measures that apply to vaccines which are reviewed by both ATAGI and PBAC before they can be listed on the NIP
26. Introduce more efficient approval processes for vaccines that value innovation and preventative health, by accepting a higher ICER threshold and incorporating the broader value considerations of vaccines – this could draw on the experience from expedited approvals of therapeutics for COVID-19
27. Align discount rates to international standards, as outlined in the Discussion Paper considered by the PBAC in 2021 – the base case discount rate used by the PBAC should be reduced to 1.5%
28. Adopt the recommendation of the Parliamentary Inquiry that the Australian Government establish a Centre for Precision Medicine and Rare Diseases within the Department of Health, which supports a nationally cohesive HTA process that is linked to state and territory Health Department efforts to prepare the health system to deliver advanced therapeutics.
29. Develop a pilot early access program with a particular focus on addressing areas of severe disease burden and high unmet need, with a preliminary assessment and ongoing involvement of the PBAC, learning the lessons from England's Cancer Drugs Fund
30. Adopt protocols for broader value considerations for HTA for advanced therapeutics and rare disease therapies, to enable equity across therapies being evaluated, with a discount rate of 1.5%
31. Establish data access arrangements and protocols to underpin managed access agreements as part of the early access pilot
32. Review access arrangements for relevant data sources, including Medicare, PBS and public hospital data, to enable more robust analysis of disease burden (including stratifying by different population groups where equity may be a concern) and post-marketing analysis, with particular focus on addressing uncertainties for rare disease
33. Adopt guidance for designing and conducting real world evidence studies for consideration by the PBAC and MSAC, aligned to guidance developed in other countries (notably, the UK and Canada)
34. Apply similar principles to all funding/HTA pathways (MSAC, PBAC and state government funding, where appropriate)
35. When TGA approval is granted, patients with high priority conditions*, such as cancer, are given immediate and affordable access to the drug or test in question. (Note that TGA approval is often fast-tracked as a result of recent changes to their processes - a paradigm that has not been significantly taken up by the PBAC, but hopefully will be considered as part of the current HTA Review). When such patients are given immediate access to the innovation in question, the PBAC, MSAC, DoH and the sponsor are then given 2-3 years to resolve whether there is sufficient evidence of cost-effectiveness to allow permanent listing on the PBS. If that can't be realised, the drug is effectively delisted and is no longer available to future patients. (*High priority conditions will need to be defined but one such definition might be 'life-threatening illnesses which are inherently unstable over time (e.g. Cancer), that generally worsen

progressively and for which treatments that work in one phase of the disease may be much less useful in subsequent phases of an illness’.)

36. Mutual recognition programs with internationals
37. Mandate collection of data with post marketing access (including equity of access) - this includes investment in clinical quality registries of current real-world data.
38. Mandate for states and territories to adopt federal ruling and eliminate variable implementation of access criteria at state level.
39. We suggest that the Government look at avenues to provide funding programs for access to drugs that are sufficiently robust for testing in the clinical trial setting, even though the area of disease research is not of interest to the company that owns the drug. Support trials that incorporate the repurposing of known cancer drugs in new indications. Australia must invest in the research infrastructure, workforce, and processes to deliver solutions.
40. Specialised therapies such as CAR T-cell therapy will require robust national data for comparative analysis versus ongoing long-term assessment.
41. HTA include engagement with clinical quality registries and a pathway for those registry entities to be invested in to provide real-time information to inform timely equitable access.
42. Improve digital support to those working on national infrastructure such as national clinical quality registries,
43. For high cost, low case numbers, especially for one off treatment modalities such as CAR T-Cell therapy there should be greater transparency over access. Nationally clinicians and patients would benefit from real-time publicly available data on deliverability to help inform understanding of equitable access. Currently do not know who didn't receive and the scope of that e.g., percentage of access per state, between states, etc...
44. The NHRA between federal and states needs to mandate implementation model(s) for approved drugs to resolve the variability and allow the drug to be supplied as intended as per the federal approval.
45. For Australia to be dynamic and responsive but retain necessary diligence and fiscal management, HTA could consider:
 - a. Provide a simplified pathway to TGA registration for novel therapeutics or technologies, e.g., if it's approved in a similar jurisdiction have process to accept that review. The example is drugs already registered in similar jurisdictions (e.g., EMA in Europe) which reduces duplication, costs, and improved efficiency.
 - b. Implement a pathway to expand the TGA labels for TGA registered drugs. Example is drug is indicted in Australia for a disease indication, and subsequently we discover a reputable agency overseas accepts its use in another indication, the TGA could add that indication label for Australia. The example is if a registration label is present in a similar jurisdiction (e.g., FDA in USA), sponsors can add that label to an already TGA approved drug without undue process or cost. Or for already TGA approved drugs, professional societies or national research groups could apply to have a label added without undue cost or complexity. These options are not unreasonable given that the majority of new drug assessment would have already occurred as

part of the initial submission to the TGA e.g., BRAF inhibitors in hairy cell leukaemia, Crizotinib in ALK+ lymphomas, bortezomib in AL amyloidosis, Rituximab in Mantle cell lymphoma maintenance.

46. Establish a pathway of conditional drug reimbursement, whereby ongoing reimbursement after a period was dependent on generation of supportive data from internationally or from Australian use. This could be best achieved via drug access linked to data acquisition via a clinical trial of national registry, with registry funding from the drug sponsor. Consideration should be made to allowing approved and registry-experienced research groups manage such registries given the established research links with the clinicians using these medicines and their data entry staff responsible for currency of data, and familiarity with collecting data pertinent to disease specific endpoints. Such registries could also capture Postcode, gender, and ethnicity of patients in these conditionally reimbursed programs to track timeliness of equity of access.
47. HTA should seek reasonable data and review from those experienced in the advancement of research in the disease area and specifically about what the limitation in the Sponsors application may mean e.g., in the ALL Blinatumomab example the limit in age range may have particular implications, and if implication is accepted by HTA then a mechanism to allow special access for clinical trials then needs to be picked up through another arrangement. The aim is to reduce burdens and increase speed to market for trials in rare, tough to treat disease such as ALL.
48. Solutions for globally accepted funded standard of care drugs such as a special access scheme for clinical trial e.g., Clinical Trial Access Scheme. Person centredness is a feature of investigator-initiated trials. The main barrier relates to routine drugs that are, in other OECD countries, not available as standard of care drugs to prescribe here in Australia. In clinical trials these standard of care drugs act as the comparator arm to new experimental treatments. The treatment for the disease that has been evidenced as best treatment is not available which limits the ability to access new, promising, clinical trial therapies for patients. A solution is to provide a mechanism for which the conduct of clinical trials for which standard of care diagnostic testing and monitoring e.g., MRD assays, PET Imaging, and therapeutic agents that are already approved and available in comparable jurisdictions can be supplied for use under clinical trial standard of care arrangement. There needs to be a pathway and price structure to address the scenario where standard of care agents are available elsewhere and a clinical trials access scheme for Australia. This would help advance new clinical trials in Australia for Australians.
49. changes to regulations and arrangements to incentivise sponsors to sell their product in Australia; For repurposing, the alternative is to allow others to have another indication registered without requiring the seller to indemnify patients against harms from their products. Both of these need Government action and changes in policy – policy must make clear that Australian Government prioritises availability and that Australians are willing to pay the price (i.e., cost / higher risk). The second may be solved by requiring international transparency on base pricing and having Australia agree to accept the base price for all registered drugs immediately from registration i.e., able to PBS subsidise at registration. Higher prices can be sought subsequently if new evidence proves them to be cost-effective at a higher price and price reductions will be required if not cost-effective at base price when there is a mandatory HTA at say 12

months post registration, with price changes flowing from 2-year point. Commercial agreement is required at entry will lay out rules for both sponsor and Government on these post-marketing price changes.

50. The HTA Review Reference committee must ensure that its recommendations align with the updated National Medicines Policy (NMP) and The Standing Committee on Health, Aged Care and Sport (the Standing Committee) inquiry report, “The New Frontier – Delivering better health for all Australians”, (the report) to ensure timely access to affordable medicines is achieved with enhanced patient/carer engagement. It is vital that the HTA review reference committee does not limit its focus to simply a ‘technical methodology’ review of HTA. Eversana encourages the Committee to also consider how enhanced patient/carer engagement will expedite faster funded access to medicines.
51. Input by patient groups and their carers should be sought early and systematically. This will allow for a more measured assessment of decision making when considering the impact of their disease and the benefit of the medicine.
52. Increase transparency for Consumer comments (including hearings and submissions). This should be described in full in the PBAC Public Summary Documents as well as how this input was used in the decision making. Patients and carers are often left in the dark about how their input adds to or informs the committee in its decision making.
53. Zimmerman Commonwealth report (2021), recommendation 5 states: “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.” Increasing the base fee for evaluation groups would benefit Commonwealth HTA agencies as it would allow more staff to work on projects and improve the quality of work due to the tight timelines of HTA submission.
54. Recommendation: to improve methodology to negotiate an effective drug price during evaluation of the first PBS submission.
55. Lower levels of evidence, expert input, clinical merit, and public consultations are essential elements in HTA assessments for rare diseases.
56. Acceptance and use of real-world evidence in throughout the review process
57. Acceptance and use of patient reported experiences.
58. Acceptance of evidence generated overseas.
59. Use of risk sharing agreements with further reviews as real world evidence emerges.
60. Horizon scanning for new technologies and therapies
61. Review of resources to deliver advanced therapies.
62. Review of supply agreements following approval with clinical input to ensure they are fit for purpose.
63. Actively involve people living with rare diseases at every stage of the HTA review process.
64. Increase the voice of people living with rare diseases.
65. Have a formal procedure for using patient reported experiences (PREMs and PROMs) in decision making and provide guidance to industry and researchers on how to use these.

66. Recommendation: The system needs to be more transparent so that it is clear how patients can provide feedback and input and how this feedback and input will be applied to the HTA system. A reformed system should provide clear and transparent information about: - how HTA decisions are made; - the role of consumers in decision making; - how consumers can be heard in the HTA process; - the pathway/mechanism for any consumer to provide their feedback.
67. Supporting systems and processes are required that ensure: - consumer representation is broad and equitable; - consumer values drive HTA; - HTA processes are subject to review and continuous improvement; - HTA processes are regularly evaluated to ensure that patient expectations are being met and that process shortcomings are iteratively addressed.; - A culture of ongoing consumer partnership and co-design.
68. HTA pathways should more readily accept evidence generated overseas.
69. There should be more risk sharing agreements for early treatments that do not have sufficient or are developing evidence.
70. MBS item numbers should be amended, where possible, rather than adding new ones.
71. Medical Research Future Fund (and other translational research funders) should co-design projects with researchers at the post award stage, so the right evidence collection and evaluations are being done from the outset.
72. HTA committees could develop a more forward-looking approach, for example by developing a roadmap of new medical services and medicines that will be critical to improving health outcomes for Australians.
73. Reframing Uncertainty as Risk Management: Instead of perceiving uncertainty as a barrier, it can be reframed as a component of risk management.
74. Better Use of Managed Entry: Expanding the utilisation of managed entry agreements can be an effective option to improve timely access. By encouraging more use of coverage with evidence development, and other similar mechanisms, the HTA system can provide earlier access to treatments while gathering real-world evidence to inform reimbursement decisions.
75. Broadening Consideration of Value and Impact: The HTA system can enhance its evaluation of value and impact by incorporating methods such as Social Return on Investment (SROI) that capture the patient experience and broader societal outcomes.
76. Incorporating Patient Insights and Lived Experience: Actively involving patients and incorporating their insights and lived experience into HTA decision-making processes is crucial. Establishing robust patient engagement initiatives, including patient advisory groups and patient-centred research, would provide valuable perspectives on the impact of treatments on patients' lives. By including patient insights throughout the HTA process, from technology assessment to reimbursement decisions, the system can better capture the patient perspective and ensure that patients' voices are heard and valued in the decision-making process.
77. Clear Pathway and Funding Architecture for Gene Therapies: Given the unique nature of gene therapies, it is essential to establish a clear pathway and funding architecture dedicated to these innovative treatments.

78. Flexible and Fit-for-Purpose Process for Genomic Technologies: To ensure the earliest possible access to genomic innovations, it is crucial to establish a flexible and fit-for-purpose process specifically designed for the evaluation of genomic technologies.
79. Encourage face-to-face negotiations and reduce the reliance on modelling parameters (as a means of setting price). In addition, defining the comparator by utilisation rather than cost will incentivise submission.
80. A further solution could be the creation of an HTA committee specifically aimed at addressing these new drugs and technologies, (a “Precision Medicine Advisory Committee”) would help solve some of these challenges.
81. Health systems aiming at improving population health given constrained resources should make efforts to ensure that decision-makers are informed (with respect to cost-effectiveness thresholds) by the strongest possible estimates (causal inference analysis) of the opportunity costs of funding decisions.
82. More funding is required for this research (into the link between patient relevant outcomes and MAUI assessed utility values) and this may be an opportunity to link PBAC data needs with MRFF funding. However, if patient-relevant outcomes are a priority, then the PBAC needs to take the initiative on obtaining funding for this research. The research should be proactive and establish a set of 'off-the shelf' links.
83. The PBAC guidance requests what are the key drivers (of the economic analysis), but it doesn't give guidance on how to quantify these drivers - that guidance should be provided.
84. Incorporate PSA into PBAC decision-making to fully enumerate uncertainty (in line with what is done by other HTA agencies).
85. Redacted models from Category 1 and 2 drugs should become available for a PBAC submission and the Category 1 PBAC recommended model should become the template for any Category 2 cost-effectiveness analyses. This is similar to what occurs for NICE in the UK, where the redacted submission is publicly available.
86. Effective marketing and advertising of the consumer process targeting the individual patient is required so patients are aware and have the knowledge of the process. Medical and policy literacy is usually devoid or limited in these populations, and a clear and plain English language is required for patients so they are aware and have the opportunity to be part of the HTA process.
87. To truly enhance the benefits of precedence in increasing efficacy by reducing procedural time and increasing the time to patient access of medicines, services and devices, the decision and the materials informing the decisions need to be transparent to be replicated in future submissions.
88. (For new technologies or those with substantial benefit) it may be possible to have an interim listing process – where PBAC has recommended the therapy, and the applicant wants to proceed based on the parameters outlined by the PBAC (such as risk sharing arrangements (RSAs), and special pricing arrangements (SPAs) etc) – where listing is highly probable but requires further negotiation. During this process, an interim listing may be possible, where the applicant agrees the interim listing would be for a specified period, and after that period (or is the applicant does not want to continue the PBS listing process), the applicant is responsible, both for supply and cost, for all “supply only” scripts for the

prescribed population during the interim listing period. This would guarantee the applicant does not prolong negotiations to sustain the status-quo of the interim period prescribing conditions, while also allowing access to therapies for patients in need. However, the Department would also need to put in place additional safeguards for the applicant – to ensure that any delays in negotiation due to the Commonwealth do not cause the timing of the interim period to expire, creating a significant risk for the Manufacturer, and a disincentive to use such a system. These safeguards could include being transparent with timelines and decisions on the Commonwealth’s side as well as mandatory extensions of deadlines if the Commonwealth does not address the required issues in time.

89. The Economically Adjusted Price (EJP), the price which the therapy is shown to be cost-effective, could be used price the therapy of the PBS, during the evidence gathering phase. After a period of time specific to the therapy, defined by the HTA Committee, the additional evidence should be used to amend the accepted cost-effectiveness model to inform the value of the therapy. The cost of data gathering should be shared – by the payer (Commonwealth) and the Manufacturer – and this method would truly be a Risk-Share Agreement.
90. While the hospital system in Australia is complex, and differs across each State and Territory, with various procurement systems, a national value-based assessment could be instituted which assists in the decision-making potential for individual health districts or other health bodies. This would assist in reducing the costs to both the States/Territories as well as the Commonwealth, while not encroaching on State/Territories management of health budgets.
91. An additional process could also be developed, where a single payer assists in the procurement of goods and services for hospitals, thus significantly increasing the purchasing power of the hospitals and reducing costs. However, such a system may be less politically palatable for the States and Territories.
92. A database linking clinical trial progress, potentially with the Australian and New Zealand Clinical Trials Registry (ANZCTR) and/or clinicaltrials.gov along with the impact to the clinical and therapeutic landscape would be beneficial for both assessment development and the Committees.
93. Office for rare diseases.
94. Platform approach to enable therapeutic development using genetic technologies.
95. HTA recognition and guidance on use of novel clinical trial designs, outcome measures, patient reported experiences for rare diseases.
96. Early engagement with researchers, people with rare diseases and clinicians to support therapeutic development for small populations/rare diseases .
 - a. Clinical referral networks and educational resources need to be established and accessible to provide diagnostic and sustainable treatment services and co-ordinate follow up for people and families with rare diseases.
 - b. These require expertise in medical genetics, neurology, and the provision of advanced therapies for rare diseases.
 - c. A coordinated strategy to collect, measure, build and translate data is needed to support quality improvement activities, monitoring of treatments and long-term outcomes, revision of clinical care guidelines.

97. Hence, it is important for objectives, policy and approaches to HTA are aligned across these different funders. This is supported in the National Health Reform Agreement.
98. Below are some practical examples of how to better include patient and/or carer perspectives in HTA decision making (experience from Sweden and the UK):
- a. Establish Patient and Carer Advisory Committees: Create formal committees consisting of patients, caregivers, and patient advocacy groups to provide input and insights during HTAs. These committees can review and comment on HTA submissions, participate in decision-making processes, and provide feedback on the patient experience. Sweden has successfully implemented patient involvement in HTAs through patient advisory committees, such as the Swedish Agency for Health Technology Assessment and Assessment of Social Services (SBU) and the Dental and Pharmaceutical Benefits Agency (TLV)¹¹.
 - b. Conduct Patient/Caregiver Surveys and Interviews: Conduct systematic surveys or interviews to gather patient and caregiver perspectives on health technologies during their evaluation. These can include questions about treatment outcomes, quality of life, and other relevant aspects. The UK's National Institute for Health and Care Excellence (NICE) engages patients and caregivers through public consultations and surveys to ensure their perspectives are considered during HTAs
99. Incorporating patient voices through robust patient engagement initiatives, including patient advocacy groups, could provide crucial insights into the effectiveness and impact of treatments, ultimately leading to more patient-centred and evidence-based decision-making.

Peak body

1. Recommendation: Where required, regulatory and legislative frameworks should be amended to ensure that the knowledge and expertise of these committee members is captured and embedded in HTA processes and methodologies.
2. Recommendation: Funding pathways must consider the whole cost of therapy, including ancillary services, to support early and equitable access to health technologies across Australian hospitals.
3. Recommendation: Introduce an accepted methodology to assess a technology's downstream cost savings to the broader health system, patients, community and the economy.
4. Recommendation: Create a flexible, ancillary process for the expedited assessment of therapies for which there is an unmet need and international evidence to support efficacy and/or cost-effectiveness.
5. Recommendation: Share Commonwealth-funded HTA assessments with state/territory jurisdictions; health system-based Drug and Therapeutics Committees (DTCs) do not have sufficient capacity to undertake cost-effective analyses independently. Sharing of assessments by TGA/PBAC/MSAC would foster streamlined and more consistent decision-making, increase equity of access to medicines nationally, reduce opportunity costs and decrease duplication of effort undertaken by individual states, healthcare services and hospitals.
6. Recommendation: Registration and reimbursement decisions should consider the use of a health technology in the acute care setting to ensure sustainable access within hospitals.

7. Recommendation: Develop a repository of non-PBS, off-label and Special Access Scheme (SAS) medicines data gathered from all hospitals across Australia to facilitate more timely decision making and provide Australians with early access to medicines needed in the acute care setting.
8. Recommendation: Address cost and time barriers prohibiting sponsors of generic medicines from applying for HTAs to expedite access to health technologies in Australia.
9. Recommendation: Applications to the Life Saving Drugs Program should not require having completed the PBAC process prior to referral. A unified/parallel process should acknowledge that ultra-rare conditions will necessarily entail highly specialised technologies.
10. Recommendation: Fit-for-purpose managed access programs should be established to ensure that real world evidence is systematically collected to address key areas of uncertainty including clinical effectiveness and PROMS/PREMS. The Consumer Evidence and Engagement unit should provide support to ensure measurements of clinical endpoints are linked with outcomes important to families.
11. Recommendation: Funding pathways for medicines used in hospitals should account for innovative, patient-centred models of care aiming to provide care to patients where they wish to receive it, without compromising medicines access and quality use of medicines.
12. Recommendation: [Horizon scanning] Establish a nationally coordinated and systematic process for identifying and monitoring emerging technologies relevant to the acute care setting.
13. Recommendation: Develop funding pathways to support the equitable delivery of innovative treatment with precision medicine to deliver personalised healthcare and better treatment options for Australians.
14. Recommendation: With respect to pharmacogenomics, applications should not be specific to particular pathology platforms/analysers; laboratories should have flexibility to choose the appropriate platforms for their services.
15. Recommendation: Collect additional data on the use of medicines across care settings, including the use of unregistered medicines and off-label medicines, to inform future funding decisions, policies, regulations and clinical guidelines preventing future medicine-related hospital admissions.
16. Recommendation: Formalise process for consumers to be involved with and comment on TGA regulatory approval.
17. Recommendation: Invest in the training of patient advocates who work alongside clinicians to enhance patient engagement and contributions to the HTA process.
18. Recommendation: The capacity of the Consumer Evidence and Engagement Unit should be increased, with funding to enhance public awareness and engagement with the unit; the unit's remit should be expanded to support HTA applications by non-commercial organisations where there is no commercial incentive for a submission.
19. Recommendation: There should be earlier engagement with consumers in all aspects of the HTA process (i.e., from the TGA to PBAC/MSAC, LSDP, etc.), to identify areas of potential uncertainty and proactively work with consumers/consumer groups to address them. Communication with patient organisations should be enhanced to ensure they are aware when sponsors first seek registration with the TGA, submit to PBAC, PBS listings are withdrawn, etc.

20. Recommendation: Consumer hearings/stakeholder meetings/engagement should be formalised as part of the HTA assessment process to ensure inclusion of patients' lived experiences when evaluating the impact of a health technology.
21. Recommendation: Amend policy/methods legislation to grant individual utility, consumer evidence, real world evidence and clinical consensus guidelines greater weight in cases where small patient population numbers lead to limited/underpowered evidence from randomised controlled trials.;
22. Recommendation: The 'value of knowing,' characterised as a "gateway to care, support, and innovation," should be applied as a principle in all HTA policies and methods.
23. Recommendation: Provide education and feedback to assist consumer organisations to understand what constitutes evidence in a HTA setting and how they can best collect this evidence, including feedback on improving the quality and effectiveness of the comments they provide.
24. Recommendation: Undertake consultation with MSAC to enhance consumer engagement in the consultation survey.
25. Recommendation: The Department of Health and Aged Care should establish HTA training and research grants to support university-based evaluation teams, enhance domestic HTA capacities and ensure the sustainability of Australia's HTA evaluation sector.
26. Recommendation: State/Commonwealth HTA processes and the National Hospital Funding Agreement should be reconciled to ensure equitable access to non-PBS/MBS items (e.g., self-funded, hospital funded, compassionate use, etc.), irrespective of health service jurisdiction.
27. Recommendation: Australia should make greater use of international approval processes to speed access to medicines already available to patients overseas.
28. Recommendation: HTAs should review the capacity of the healthcare system, particularly the hospital sector, to deliver the technology being assessed.
29. Recommendation: Expedite the submission process by providing clearer direction to sponsors on what specific data required by PBAC/MSAC and reduce the provision of unnecessary information by sponsors. [SHPA;]. Streamlined application processes should be introduced for approved technologies seeking new/expanded indications/applications.
30. Recommendation: Evaluation of first and second generic versions of innovator medicines should be prioritised; fees for the evaluation of generic versions of medicines known to often be in shortage or limited supply should be waived.
31. Recommendation: Establish a fund to support (non-industry) health professionals, peak bodies and consumer groups to sponsor registration and reimbursement applications for certain health technologies.
32. Recommendation: Increase funding to support domestic clinical trial capacities.
33. Recommendation: HTA processes should be updated to recognise contemporary and emerging models of care, including hospital in the home, hospital in the nursing home, pharmacist-led outpatient clinics, aged care outreach programs, digital health and other emerging models of care.
34. Recommendation: The MBS Review Pathology Clinical Committee Recommendations should be adopted to ensure appropriate fees (and comparator values) for current and emerging pathology tests.

35. Recommendation: Alignment between MSAC and PBAC should be enhanced; the recommendation of the Standing Committee should be adopted: “The Australian Government ensure the membership of the MBAC and MSAC provides the appropriate expertise...This should include ... enhanced cross-membership between the two committees.”
36. Recommendation: Introduce specialist HTA groups [e.g., for genomics].
37. Recommendation: Clinical trial guidelines should require consumer codesign and include PREMS and PROMS.
38. Recommendation: Reduce incentives to cost-shift by expediting MSAC/PBAC applications for tests provided in the NHRA-funded sector.
39. Recommendation: Align NHRA and MBS/PBS fees to reduce perverse incentives to cost-shift.
40. Recommendation: Provide a list of alternate funding arrangements that could potentially be used (i.e., other than via the NHRA or PBS/MBS) when applications are made.
41. Recommendation: Provide PBS funding for hospital in-patient prescribing
42. Recommendation: Develop a single-funder model for health technologies provided in hospitals to [reduce duplication in the assessment of safety and cost-effectiveness and] facilitate early and equitable access to high-cost and complex medications
43. Recommendation: Enable public hospital pharmacies to supply PBS-subsidised medicines for public hospital inpatients to achieve equity and enhance quality use of medicines and medicines safety.
44. Recommendation: Create a national approach for in-hospital use of medicines
45. Recommendation: Enable hospital pharmacists to supply medicines to Indigenous Australians under Closing the Gap PBS Co-Payment Measure.

Jurisdictions

1. Recommendations to the Review:
 - a. Consult with the Health Technology and Genomic Collaboration to align with planned implementation of the NHRA 2020-25 long-term reform to develop a nationally cohesive approach to Health Technology Assessment.
 - b. Accelerated access should be supported with a robust monitoring and evaluation framework as a shared responsibility between industry and governments, with a commitment to disinvest if outcomes do not meet expectations.
 - c. Consider the cost benefit of HSTs and longer-term sustainability in the HTA methodology for both Commonwealth and State governments. As part of this analysis, comprehensive reporting requirements for states and territories should be considered in the health system service delivery costs.
 - d. Examine whether existing HTA pathways can be simplified to manage the range of novel therapies that will be seeking market access in the short to long term.
 - e. Strengthen the shared responsibility between industry, research/academia and government to deliver affordable medicines for long term sustainability. This includes support for locally

developed therapies, especially where a public hospital, university, medical research institute, or SME is the sponsor.

- f. Consider elements of the New Zealand Pharmac system for adaption in Australia to support longer term sustainability and competitive pricing.
- g. Strengthen patient experience requirements in HTAs to inform decision making and implementation. Requirements for supporting research may include qualitative interviews or patient surveys beyond the traditional validated tools used for research.

2. Key Recommendations:

- a. All MSAC and PBAC submissions and assessment reports should be published and not restricted by commercial-in-confidence. The evidence-base and assumptions related to decision-making should be transparent to all stakeholders and will assist in local implementation.
- b. S &Ts should be able to provide input to HSTs assessed by the PBAC (for example, Zolgensma (onasemnogene abeparvovec) for spinal muscular atrophy)
- c. Sharing of local clinical and economic data as relevant with local and state HTA committees may be very helpful, as unlike PBAC applications, applications to local and state HTA committees is mostly clinician-driven.
- d. Early access to and funding of novel therapies should be supported by adequate data collection. For example, in the UK the NHS has a range of registries and databases for outcomes collection:
 - i. For leadless pacemakers “Clinicians should enter details about all patients having leadless cardiac pacemaker implantation for bradyarrhythmias onto the National Institute for Cardiovascular Outcomes Research database and review local clinical outcomes” (<https://www.nice.org.uk/guidance/ipg626/chapter/1-Recommendations>).
 - ii. The National Cancer Registration and Analysis Service (NCRAS) in the UK (<https://www.cancerdata.nhs.uk/>) o NHS Digital clinical audits and registries (<https://digital.nhs.uk/data-and-information/clinical-audits-and-registries>). This usage and clinical data should be shared with relevant stakeholders (e.g., state and territories, clinicians), rather than being collected under commercial-in-confidence agreements. These arrangements should be part of the initial approval and funding agreement.
- e. For novel therapies approved for early access, there should be an initial conditional time-limited approval and funding. Ongoing funding should be based on defined criteria.

- 3. A horizon scanning activity, as previously undertaken in Australia by HealthPACT, or as undertaken by the NIHR Innovation Observatory in the UK. This activity would provide local stakeholders with valuable information on new and emerging therapies and enable a planned assessment of new technologies based on evidence and local needs. Subsequent to the Solomon review, the Health Technology Opportunity Scan provided to AHMAC by NSW Health in December 2017 (the Menzies review, provided to AHMAC at their meeting on 8 December 2017) provides options for a nationally cohesive approach to HTA. This approach would ensure effective cooperation across all jurisdictions and reduce duplication of work and includes a horizon scanning activity as an important method to actively identify new and emerging therapies.

4. Improved collaboration between PBAC and HTA state and hospital committees: We would like to establish a relevant and specific communication channel and require when necessary fee-free state initiated submissions when PBAC listing restrictions do not reflect current clinical guidelines (e.g. dose optimisation of infliximab in inflammatory bowel diseases, or when a medicine is recommended in international guidelines but unlikely to be TGA-approved or PBS-funded (e.g. delamanid in multi-resistant tuberculosis) or when an application was rejected by PBAC and the company did not reapply.
5. Improved communication with consumers and health professionals of the PBAC decisions, explaining in a consumer-friendly language the reasons why a medicine or therapy has been recommended or rejected for listing.
6. For funding of HST:
 - a. Split and cap payments, to mitigate risk due to the immature evidence and uncertainties in therapeutic outcomes, costs and patient numbers.
 - b. Follow-up review of HSTs approved under the NHRA.
7. Establish an overarching Office that can assist in ensuring that applications are directed to the appropriate (HTA) assessment entity. The existence of such an Office, with support and input from the States and Territories, would ensure correct referrals to prevent delay and bring medicines, therapies, and products to patients within reasonable timeframes.
8. PBAC should be able to revisit some previous recommendations when a medicine become funded in many other countries. Otherwise, we are potentially (1) disallowing patients access to these medicines or (2) leaving each individual hospital to decide and pay higher prices without the bargaining power of PBAC. There is a need for a more flexible approach for PBAC to work with sponsors to find a pathway to market for these medicines rather than leaving them to decide whether to make a resubmission knowing that they can instead pursue hospitals and supply to drug at a higher price. There is a need for a nationally coordinated HTA approach for these medicines that hospitals are funding rather than each hospital repeating the process. This approach should include liaising with PBAC on a regular and ad hoc basis.
9. Need to better use hospital-based or national rare diseases datasets to measure usage and outcomes. Recommendations for post-market reviews should also consider use of high-cost medicines that may not be or only partially PBS-funded but may be used by public hospitals based on their efficacy in rare clinical conditions or when a PBS submission has never been sent by the sponsor.
10. There needs to be an agreed approach to the use of surrogate markers which takes a patient centred approach and considers not only the possible benefits of treatment but also the financial and emotional burden placed on, predominantly cancer patients.
11. Nationally cohesive HTA, as indicated in the NHRA, would be highly beneficial to assess new, high-cost therapies. This would provide public patients with access to therapies not otherwise assessed through PBAC or MSAC - the newly established Health Technology and Genomics Collaboration will play an important role in this area
12. Imperative that all HTA processes and decisions are not just open to the Sponsor but are equally open to both the clinical and general community.

13. Newer health technologies may require changes to the review criteria used to assess positive recommendation. As new technologies allow for the decreasing, or eliminating of, medical costs for one group of patients those freed up costs should be considered for reinvestment opportunity to other patient groups whose clinical needs continue to be unmet by the market.
14. Pricing treatments should allow health systems to reward innovation and improve access for to all patients.
15. A specific consideration for HSTs would be to enable delivery in private hospitals to support broader access and develop mechanisms to recognise and account for patients using private health insurance in block funding arrangements for public hospitals. Over half the Australian population has private health insurance, and patients should have a right to use this as part of their treatment (e.g., choosing their specialist). Permitting the use of private health insurance, while ensuring the appropriate mechanisms are in place to recognise and account for these instances in the average costings, will likely result in improved consumer confidence and potential cost savings for these high-cost therapies.
16. Clear pharmacovigilance plans need to be in place for monitoring safety of new therapies.
17. It would be preferable to have a dedicated pathway for the assessment of cell and gene therapies, which can incorporate the expertise of relevant medical specialties such as immunology, haematology, and genetics. The present system results in various genetic therapies being considered by differing entities on a case-by-case basis, resulting in confusion on behalf of the sponsor, with differing public funding and reimbursement pathways. Additionally, there is potential that a cell or gene therapy could be assigned to either entity based on delivery location (i.e., inpatient or outpatient), but this could potentially change (e.g., originally delivered on an inpatient basis as a new treatment, but after some time has elapsed and clinician confidence has grown could be then given on an outpatient basis, which would alter the correct reimbursement pathway).

Other

1. Create more appropriate commercial incentives for business to invest in translational research.
2. Coordinate government funding and co-funding for integrated and translational research
3. Reduce regulatory barriers for translational research.
4. Support more efficient clinical trials, which can often be the most expensive and resource consuming part of translation.
5. Design new approaches to monitoring safety and effectiveness of new treatments.
6. As artificial intelligence (AI) is playing an increasing role in the development of new health technologies, ACD strongly recommends that the review of HTA policy and methods incorporates expertise from those specialties in which we are seeing rapid advancements in AI.
7. ACD recommends that a dermatologist with expertise in AI is involved in the HTA policy and methods review to provide advice on the incorporation and assessment of new health technologies in dermatology utilising AI.
8. Addressing current perceptions and developing standardised tools/processes to incorporate patient insights in an equitable and transparent manner are recommended.

9. All approvals are “disease-based”. A more 'person-centred' approach would be to have drugs approved that are based on the molecular abnormality the patient's cancer harboured. A classic example would be BRAF mutations.
10. Facilitate a process for applications by non-pharma entities.
11. While there have been opportunities for stakeholder input into PBS submissions, this process can be improved by actively seeking out comments from stakeholders with relevant expertise. Opinions need to be weighted according to the level of the expertise, i.e., patients vs. pt advocate vs. health care professionals vs. subspecialists in the area in question.
12. Seek out guidance from peak bodies, such as Myeloma Australia's Medical and Scientific Advisory Group, rather than relying on voluntary contributions and the evidence provided by industry.
13. Use Australian data. For example, we have the Myeloma and Related Diseases Registry data on more than 4000 newly diagnosed patients. This dataset represents the only comprehensive real-world evidence on outcomes for myeloma in Australia.
14. Invest in educating the public about this process so that individuals, including patients, can engage in an efficient and impactful manner.
15. Regularly seek feedback on the HTA process to identify issues as they arise.
16. Increase the number of streamlined applications that allow non-industry groups to seek access for multiple disease areas in one submission.
17. Uncouple clinical approval from financial approval and make this separation clear.
18. Involve the “relevant” health care opinion leaders into the decision making rather than “representative stake holders”. HTA needs to know who those relevant stake holders are, not only at a national level, but at the hospital level if the decision pertains to a health provision that are restricted to certain hospitals.
19. Look to the example set by Myeloma Australia to educate and advocate for the inclusion of those impacted by Myeloma in PBAC and MSAC processes. We have developed a system of education, communication and advocacy that helps our community to interact with the Australian HTA system as it is.
20. Recognise and invest in those groups doing this work for free or provide a system to relieve this burden.
21. ANDHealth advocates that the government consider broadening its definition of health technologies to include digital health and provide increased levels of support for SMEs to access the HTA system. Advances like preventative and personalised medicine, connected point-of-care diagnostics, medication management and adherence, patient engagement, remote rehabilitation and remote patient monitoring are the future of the health technology sector. A wider scope for health technologies under the HTA process, would include all of these options.
22. Establish a delinked, subscription model pilot fund for novel antimicrobials: AAMRNet strongly recommends the Australian Government invest in the rapid establishment of its own fit-for-purpose pilot fund, taking learnings from the UK pilot. The fund could be jointly supported by the Federal, and State and Territory Governments. Doing so would deliver on a key recommendation of the Parliamentary report, The New Frontier – Delivering better health for all Australians. It would also be a positive step toward delivering on Australia's National AMR Strategy and global calls to action from the G7. Most importantly, it would help

protect Australia's health security by ensuring Australians have equitable and timely access to life-saving antibiotics for infections that could not otherwise be treated with existing medicines.

23. Review clinical trial requirements for novel antimicrobials.
24. Develop fit-for-purpose HTA pathways for antimicrobial medicines.
25. Reform existing approaches to value vaccines as part of a review of the National Immunisation Program.
26. Develop a clear policy framework for the regulation and reimbursement of point-of-care diagnostic tests.
27. Strengthen existing, and introduce additional, flexible data and market exclusivity extension options for novel antimicrobials.
28. More stakeholders in independent specialities who can help generate, score, validate, record and utilize IT or use their biggest capital - experience, to avoid unclear scenarios... and be included right from the initial iterative phases.
29. Clinical trial access in terms of disease modifying treatments should be increased.
30. HTA should support evolving expertise in consumer partnership in Australia so that we can fully draw on all the benefits - even the low literate consumer can advise on readability of information- but we need to have deeper conversations about what is ethical, risk/benefit reality, patient as opposed caregiver perspective, what constitutes a quality life without condemning patients to living under a microscope if the benefit is unclear.