Medicines Australia

DRIVING HTA REFORM: A SUMMARY OF LEARNING FROM THE HTA ROUNDTABLE

NOVEMBER 2024

ACKNOWLEDGEMENT OF COUNTRY

We acknowledge the traditional custodians of the lands on which we research and work, and we pay our respects to the Elders past, present and future. We recognise and respect their cultural heritage, beliefs and continuous relationship with the land.

ACKNOWLEDGEMENT

This report presents findings and recommendations derived from a roundtable involving patients, industry, and academic stakeholders. Medicines Australia would like to thank **HT**ANALYSTS for managing the roundtable, and Pfizer Australia for hosting the event.

We are deeply grateful to everyone who participated in the roundtable, especially the patients who gave up their time and lived experience. Our aim is for these findings to pave the way for reform to ensure patients have rapid and equitable access to new treatments.

HTANALYSTS has been providing boutique value, impact and policy assessment services for over two decades. Originally founded in 2002, our organisation has grown to become a leader in social impact consulting, providing services to the healthcare industry and beyond.

OUR PURPOSE IS TO HAVE A POWERFUL IMPACT ON THE HEALTH OF SOCIETY BY CONNECTING PEOPLE WITH THE BEST TREATMENTS IN THE FASTEST AMOUNT OF TIME.

Medicines Australia | Better health through research and innovation

CONTENTS

ABBREVIATIONS

CF	Cystic fibrosis
DoHAC	Department of Health and Aged Care
FDA	Food and Drug Administration
EMA	European Medicines Agency
HTA	Health Technology Assessment
HUCN	High unmet clinical need
ICER	Incremental cost effectiveness ratio
LSDP	Life-saving drug program
LTFU	Loss to follow up
MA	Medicines Australia
MAP	Managed Access Program
MP	Member of Parliament
MSAC	Medical Services Advisory Committee
NSCLC	Non–small cell lung cancer
OECD	Organisation for Economic Co-operation and Development
PBAC	Pharmaceutical Benefits Advisory Committee
PBS	Pharmaceutical Benefits Schedule
PSD	Public summary document
PICO	Population, Intervention, Comparator, Outcomes
PSD	Public Summary Document
RWD	Real world data
RWE	Real world evidence
TGA	Therapeutic Goods Administration
QALY	Quality adjusted life year
QoL	Quality of life

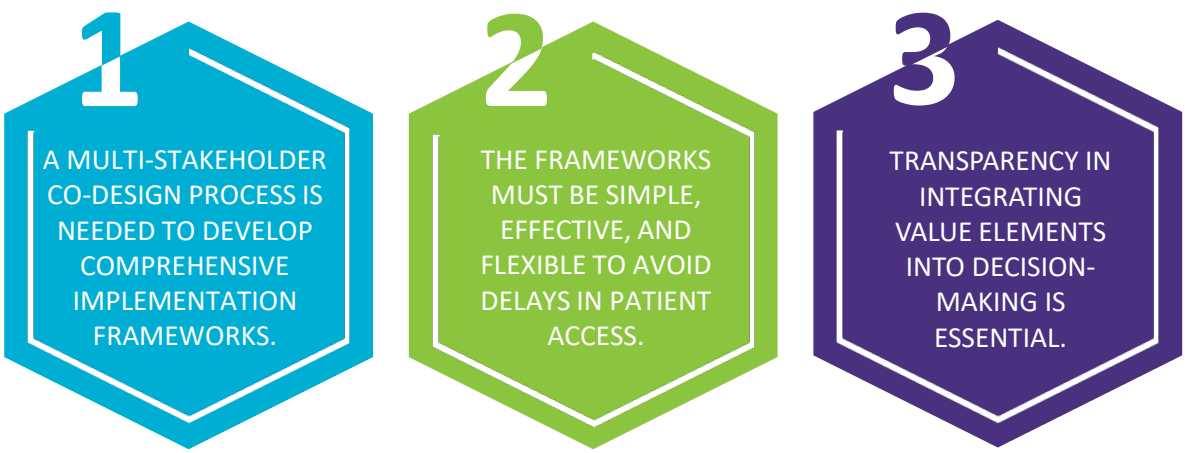
EXECUTIVE SUMMARY

The Federal Government has released the final report of the Health Technology Assessment (HTA) Review, marking the first comprehensive review of Australia's HTA system in nearly 30 years.

Prior to the report's release, Medicines Australia convened a multi-stakeholder roundtable on 26 July 2024 to explore three transformative topics from the HTA Options Paper: the development of a qualitative value framework, the establishment of a bridging fund, and the use of managed entry. These topics were chosen because, together, they have the potential to help Australia realise the vision of equitable access to the latest medical technologies within 60 days of TGA registration. The objective was to explore the topics and propose frameworks for implementation.

During the roundtable event, topics were first introduced by leading experts, who outlined the overarching principles and concepts. Attendees were then divided into three groups, each discussing one of the selected topics. A series of subsequent virtual roundtables and interviews were conducted using a similar approach.

KEY FINDINGS



QUALITATIVE VALUE FRAMEWORK

A focus of the discussions was the development of a qualitative value framework that can be integrated into the HTA decision-making processes. The key insights included:

- **Stakeholder collaboration:** There is a need to form a working group with key stakeholders to define and align on the purpose and goals of the value framework.
- **Value elements:** The value elements must be clearly defined, transparent, and consistently integrated into decision-making processes. However, the method for selecting these value elements remains a subject of debate.

BRIDGING FUND

The establishment of a bridging fund to enable early access to innovative therapies was seen as a significant enabler for HTA reform. The discussions revealed:

- **Structure and source of funding:** There needs to be alignment on both the structure and sources of funding to ensure long-term sustainability and accessibility.
- **An appropriate model**: Stakeholders must come together to develop a model that clearly defines the structure, timing and eligibility criteria, noting there is experience from similar funds overseas that can be leveraged.

MANAGED ENTRY

Managed entry, although available in the current HTA process, is underutilised and often misunderstood. Stakeholders agreed that:

- **Trust and co-design:** There needs to be trust between stakeholders, and a revised framework should be developed through co-design.
- **Reframing managed entry:** Managed entry could be reframed as ‘provisional access’ to better reflect its purpose.
- **Increased utilisation:** A revised approach could facilitate broader acceptance and usage of managed entry agreements.

CONCLUSION

The roundtable discussions highlighted the importance of engaging a broad range of stakeholders to develop comprehensive and effective solutions. However, participants also recognised the need to balance this collaborative approach with the urgency of implementing reforms. While there was almost unanimous agreement on the necessity of reform, the specifics of these reforms remain open to debate. Finalising these details will require continued consultation, and it was recognised that the processes themselves can also evolve and improve over time.

NEXT STEPS

Medicines Australia has outlined the following next steps to maintain momentum for implementation of HTA reform following the release of this report and the HTA Review:

HTA REFORM POLICY ROUNDTABLE —BACKGROUND, INTRODUCTION AND METHODS

INTRODUCTION

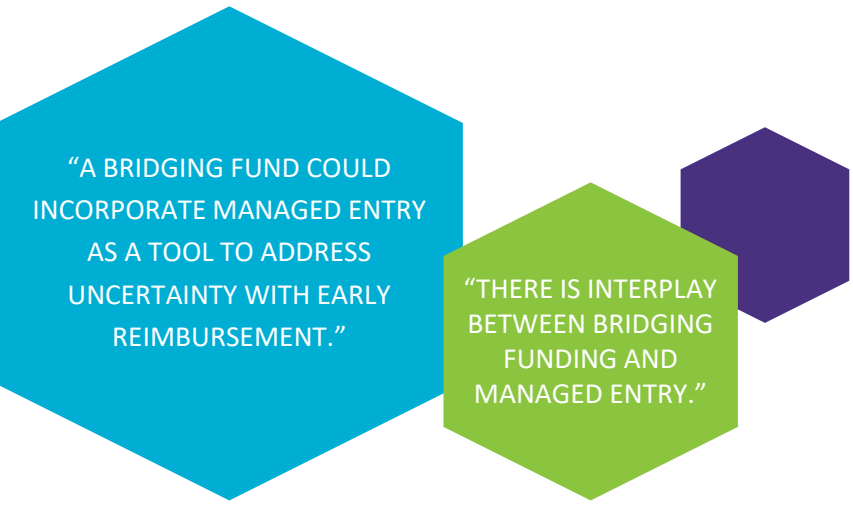
BACKGROUND

As part of the HTA Policy and Methods Review, the Reference Committee developed an Options Paper for public consultation in January 2024, containing a set of draft options for reform of Australia's HTA system.[1] The Reference Committee then reconsidered the options following a second round of consultation and arrived at a final report which was published in September 2024. At the time of the Medicines Australia Roundtable, the final report had not been released.

Medicines Australia chose the following three topics from the Options Paper to be explored through a series of discussions, including a multi-stakeholder roundtable event and additional conversations with the patient community:

1. QUALITATIVE VALUE FRAMEWORK
2. BRIDGING FUND
3. MANAGED ENTRY

These topics were selected by Medicines Australia because they represent some of the most promising options with the potential to drive transformative change. Together, they can help achieve the vision of all Australians having access to the latest medical technologies within 60 days of TGA registration.



HOW THIS REPORT WAS DEVELOPED

This report was informed by a series of discussions facilitated by Medicines Australia, including a roundtable event, two online forums, and 1:1 interviews.

On 26 July 2024, a multi-stakeholder roundtable event was held in Sydney to discuss three topics: development of a qualitative value framework, the establishment of a bridging fund and the use of managed entry.

The roundtable comprised representatives from industry (18), academia (3), clinicians (2) and the patient community (8). Participants were divided into groups representing each of the chosen topics. Over two breakout sessions, groups were asked to develop responses to the following questions and topics:

- What do stakeholders want from this?
- What are the barriers?
- What are the enablers?
- What is needed to achieve success?
- Prioritisation of activities
- Action plan

Breakout groups presented their findings back to the wider group to stimulate further discussion.

Following the roundtable event, broader input was sought from the patient community and clinicians via online forums and 1:1 interviews. Participants were given the opportunity to provide feedback and answers to the same questions posed at the roundtable event.

The input and ideas generated from these events has been synthesised to inform this report. Participants were given the opportunity to review the findings and provide feedback prior to publication.

THE HTA REVIEW

Qualitative Value framework: A transparent explicit qualitative framework to define value, enhance equity and guide decision making.

Bridging funding: A special technology agnostic funding program that will allow for interim patient access in a reasonable timeframe, which is separate from standard funding pathways.

Managed entry: A conditional arrangement between a manufacturer and payer that enables earlier reimbursement of a health technology with mechanisms to address uncertainty with regards to performance or utilisation.

Real world data/evidence (RWD/RWE): RWD refers to information collected outside of traditional clinical trials, often from sources like electronic health records, patient registries, and health apps. RWE is the clinical insight derived from analysing this RWD, providing a broader understanding of health outcomes, treatment benefits, and potential risks in real-world settings.

"THE HTA SPACE IS FULL OF TERMINOLOGY AND COMPLEXITY. IT IS IMPORTANT WE ARE ON THE SAME PAGE."

WHY IS IT IMPORTANT

The importance of this review, particularly from the patient perspective, can be summarised into four areas:

TIME TO ACCESS

- This is a key area, particularly when there is high unmet clinical need
- We need equity across society and affordable care for all Australians

EVIDENCE

- Patients are often surprised by what is not included in the evidence
- We need to think about all the things that matter in the patients' lives, beyond what is captured in data

BELONGING

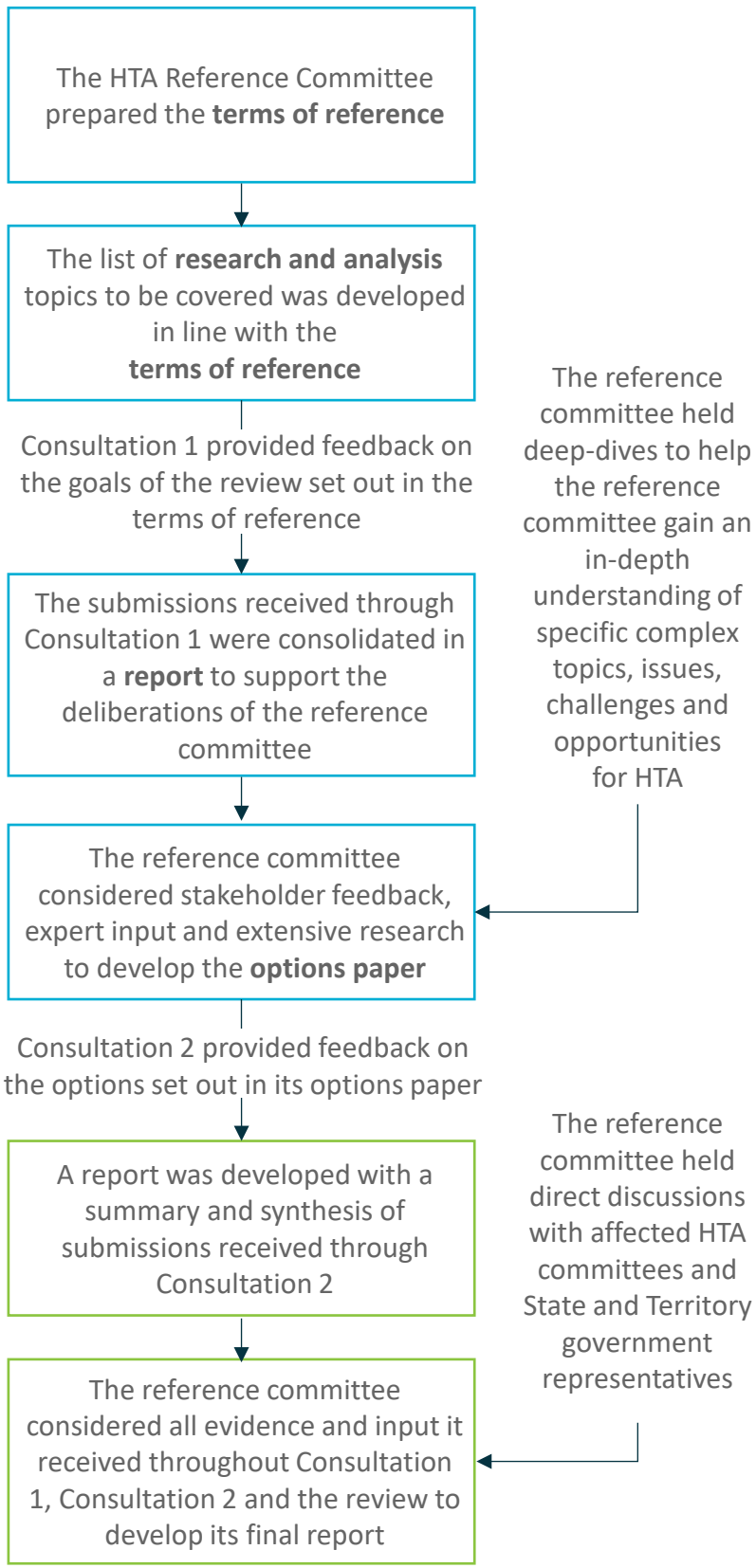
- The community wants to be part of decision making
- There is an opportunity for this to become something that belongs to all of us
- There needs to be co-design

TRANSPARENCY

- How do we show people how/what we consider in the evidence weigh up?
- We need a more transparent system so that we can have a more informed interaction

HTA REVIEW PROCESS OVERVIEW

THE HTA REVIEW RECEIVED INPUT FROM STAKEHOLDERS THROUGH VARIOUS CONSULTATION ACTIVITIES



REAL WORLD DATA AND REAL-WORLD EVIDENCE

Real world evidence (RWE) plays an important role in decision making in HTA, perhaps now more than ever. **Real world data (RWD) and the evidence it generates underpins each of the three chosen topics (qualitative value framework, managed entry and bridging fund), and is critical to their success.**

The HTA Review was informed by a series of research and analysis papers, including a paper on RWD and RWE.[2]

"OPTIMISING THE AVAILABILITY AND USE OF REAL-WORLD DATA AND REAL-WORLD EVIDENCE TO SUPPORT HEALTH TECHNOLOGY ASSESSMENT IN AUSTRALIA"

The paper addressed two interconnected parts:

1. RWD AVAILABILITY AND ACCESS, AND THE OPPORTUNITIES FOR USE IN HTA

2. A ROADMAP FOR OPTIMISING THE AVAILABILITY AND USE OF RWD TO GENERATE RWE IN HTA

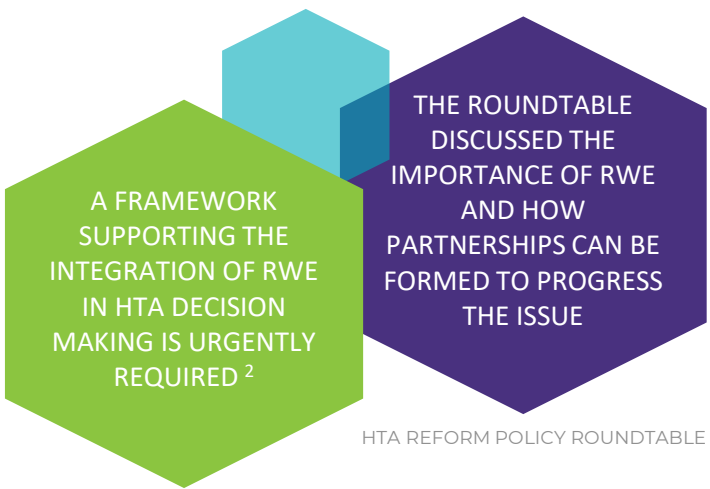
The paper recognised that RWD and RWE play an important role across the entire HTA pipeline, from pre-market to post-market. These roles include but are not limited to epidemiological estimates, determining eligibility criteria and patient selection, safety and effectiveness monitoring, and real-world utilisation and cost-effectiveness.

The paper identified several barriers and enablers of access to RWD for HTA, as well as the sources and types of RWD needed, and the opportunities to optimise its availability and use.

Opportunities to maximise the value of RWD and RWE in HTA were presented as being underpinned by four interconnected principles:

PARTNERSHIPS | TRUST | DATA INFRASTRUCTURE | METHODS

A roadmap to support these principles, including a series of steps for immediate and longer-term implementation was presented in the second part of the paper.[2]



QUALITATIVE VALUE FRAMEWORK

AN EXPLICIT TRANSPARENT FRAMEWORK TO DEFINE VALUE, ENHANCE EQUITY AND GUIDE DECISION MAKING IN HTA

DEVELOPMENT OF A QUALITATIVE VALUE FRAMEWORK

A brief background on value frameworks in the Australian HTA context is provided below.

HISTORY IN HTA

Value frameworks have existed in HTA decision making (such as PBAC decision criteria), however not in the explicit form proposed in this report. There is currently **no clear process** for **how these value elements are quantified** or used to aid decision making.

VALUE FRAMEWORK

PBAC recommendations are based on:

Quantitative factors: comparative health gain and cost-effectiveness, patient affordability in the absence of PBS subsidy, predicted use and financial implications for the PBS and the health budget.

Less quantifiable factors: overall confidence in the evidence, equity, presence of alternatives, severity, ability to target therapy, public health issues and any other relevant factors.

IMPLICIT VS EXPLICIT

Value elements in the current PBAC guidelines are implicit, therefore the impact they have on decision making is not transparent.

Value elements need to be explicit to improve transparency in decision making and allow for enhanced communication.

THE AIM IS TO PROVIDE CLARITY, TRANSPARENCY AND CONSISTENCY REGARDING HOW VALUE ELEMENTS (OTHER THAN CLINICAL AND COST-EFFECTIVENESS) ARE FACTORED INTO DECISION MAKING.

QUALITATIVE VALUE FRAMEWORK OPTIONS

The HTA Review Options Paper presented several options relating to the development of a value framework.

OPTIONS PRESENTED IN THE HTA REVIEW OPTIONS PAPER

In consultation with stakeholders, the HTA Committee should develop **explicit guidance regarding value elements** (beyond clinical effectiveness, cost-effectiveness, and financial impact).

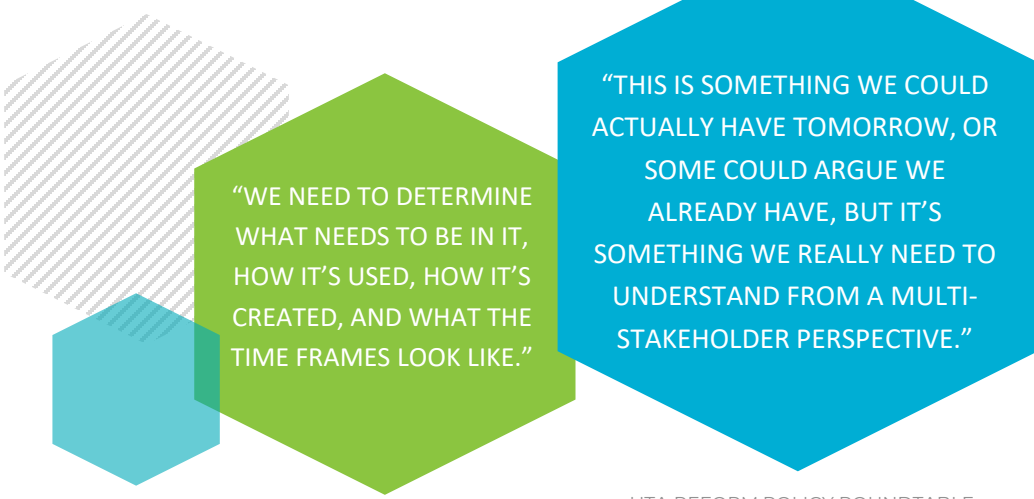
Aim is to adopt a patient-centric approach and provide greater confidence that the committee is considering factors that are of value to both patients and society.

A framework should be informed by published research and public consultation. Recommend developing a checklist to assist HTA decision makers to integrate equity considerations into their deliberations in a more comprehensive and systematic way.

What are the value elements?
How will they be considered?
How can Sponsors provide additional data to address value domains?
What impact will they have on decision-making?
How are First Nations Peoples impacted?

Framework should allow enough flexibility for the deliberation process itself to add value to the decisions i.e. not be pre-weighted and scored.

The consideration of the value elements would need to be explicit before, during and after consideration of a technology and be transparently communicated in Public Summary Documents.



LEARNINGS FROM THE DISCUSSIONS

WHAT DO STAKEHOLDERS WANT FROM THIS?

Developing a value framework needs to be an inclusive process involving all relevant stakeholders. The whole community needs to come to agreement on what the explicit value elements should be, and how they will be considered in HTA.

The framework needs to be simple, effective, transparent, agile and flexible, while not diverting resources or causing delays to patient access.

KEY THEMES:

BROAD CONSULTATION
EXPLICIT VALUES
TRANSPARENCY
FLEXIBILITY
MINIMISE COMPLEXITY

WHAT ARE THE BARRIERS?

Several barriers were identified, such as methodological barriers, and the method used to design and implement the framework. Applying qualitative elements of value quantitatively is inherently difficult, which can create perceived uncertainty in the process and act as a barrier to implementation.

"THERE NEEDS TO BE ALIGNMENT ON WHAT A VALUE FRAMEWORK IS AND WHAT PROBLEM WE ARE SOLVING."

WHAT ARE THE ENABLERS?

The HTA review is an important enabler, providing an opportunity to initiate change and develop a roadmap to implementation.

There is an abundance of research in this space, including multiple examples from HTA bodies overseas that can be leveraged.

Note: These learnings from the breakout discussions are summaries informed by the ideas presented during the multi-stakeholder discussions, which are presented in full for each topic in the appendix.

"WE CAN'T LET ADDITIONAL COMPLEX ANALYSIS SLOW DOWN TIME TO ACCESS – PERHAPS WE COULD AIM FOR EARLY AGREEMENT ON THE VALUE ELEMENTS SPECIFIC TO THE PRODUCT."

- We need to consider to what extent these value elements reflect the Australian taxpayer. Who is the priority? Is it the patient (today) or the taxpayer (tomorrow)?
- Does the value framework belong in legislation or somewhere else entirely?
- How does a values framework play into real world evidence collection?

LEARNINGS FROM THE DISCUSSIONS

KEY THEMES:

EARLY AGREEMENT
INCLUSIVE
CO-DESIGN
EQUITY

"IN TERMS OF TIME FRAMES, WE NEED TO START ADVOCATING FOR THIS NOW, WE DON'T NEED TO WAIT FOR THE FINAL HTA REPORT TO BE RELEASED."

"THIS IS VALUABLE AND IMPORTANT. IT IS OUR JOB TO TRANSLATE THE VALUE OF THIS FRAMEWORK TO DECISION MAKERS."

"THESE IDEAS NEED TO BE SOCIALISED, WE NEED BUY-IN AND MULTIPLE PERSPECTIVES ON THIS."

WHAT IS NEEDED TO ACHIEVE SUCCESS?

Achieving success requires getting everyone on the same page. This involves defining the problem, the purpose and the elements that should be included.

There should be early agreement on what the value elements are (specific to the product), as to not slow down the HTA process. Feedback and transparency on how elements have informed decision making is essential.

PRIORITISATION OF ACTIVITIES

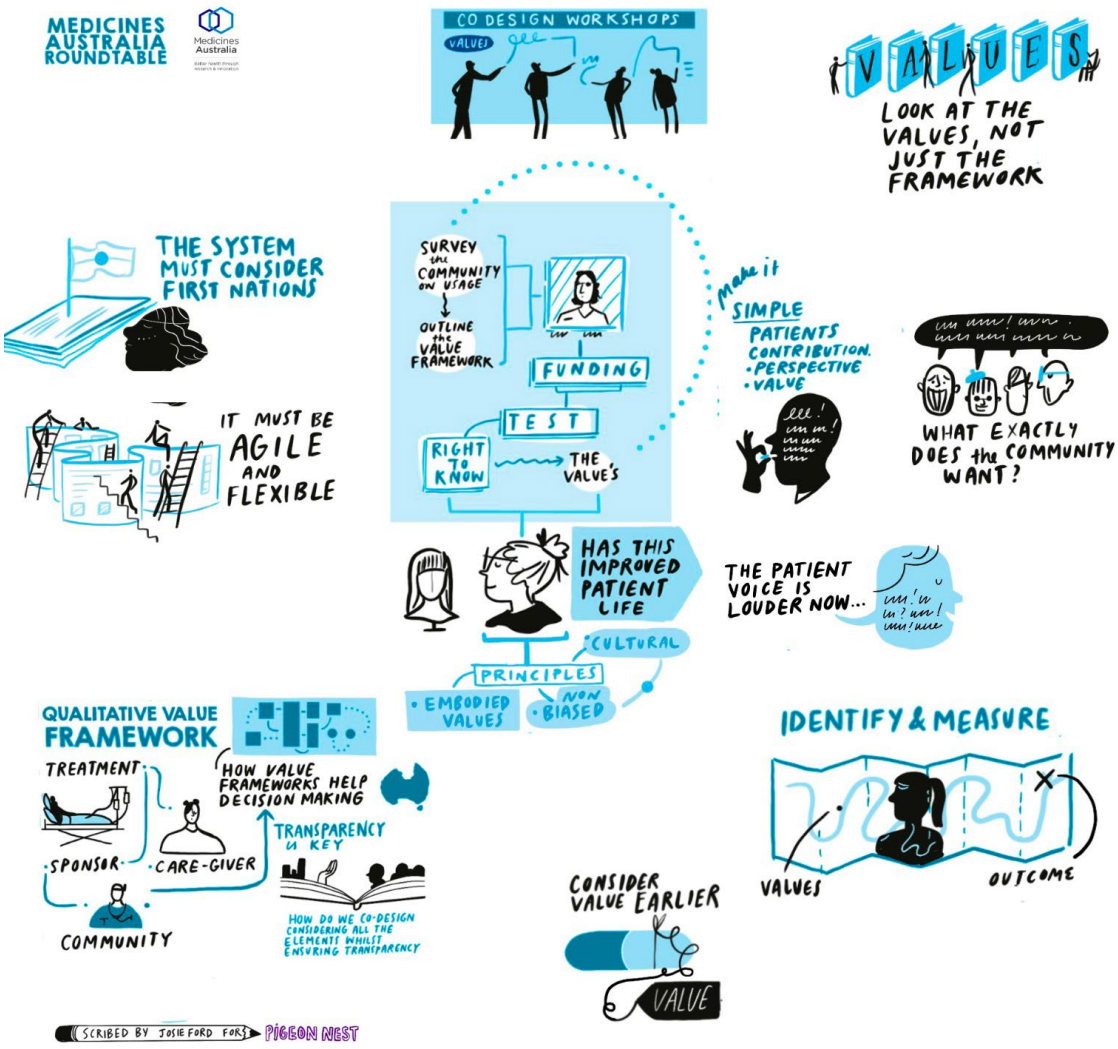
A stakeholder workshop and consultation process was suggested to develop a list of value elements and determine how they will be measured, validated and used to inform decision making. The consultation process needs to include a broad range of stakeholders that represents the users of medicines and could even extend to the Australian taxpayer.

ACTION PLAN

The critical step in developing a value framework is to advocate for a broad co-design process and develop a multi-stakeholder proposal.

- Do all the value elements need to feed into cost-effectiveness? Could this sit outside cost-effectiveness and be complementary?
- We need to consider if this is for PBAC decision making, or to reflect Australian public values?
- We need to consider why/how we implement this. Will this framework allow for a path to broad or equitable access?

VALUE FRAMEWORK



INSIGHTS

Incorporating a qualitative value framework into HTA is widely recognised as crucial for ensuring that the societal value of a health technology is fully considered in decision-making. To advance this initiative, the first step is to convene a diverse group of stakeholders to align on the definition, purpose, and goals of a value framework.

A simple and effective value framework is essential to avoid delays in patient access due to overly complex analyses. Leveraging existing research and global examples of value frameworks will help speed up the process. The current PBAC decision criteria are also a reasonable starting point to inform discussions.

Value elements, whether quantitative or qualitative, must be explicit and transparently integrated into decision-making. Agreeing on product-specific value elements early in the HTA process will help streamline assessments. The HTA Review is a vital enabler of the value framework and presents an opportunity to move beyond the current narrow definition of value embedded in legislation.

BRIDGING FUND

A SPECIAL TECHNOLOGY-AGNOSTIC FUNDING PROGRAM THAT WILL ALLOW FOR INTERIM PATIENT ACCESS IN A REASONABLE TIMEFRAME, WHICH IS SEPARATE FROM STANDARD FUNDING PATHWAYS

ESTABLISH A BRIDGING FUND

The bridging fund would be a technology- agnostic funding program that is separate from standard funding pathways. A brief background on bridging funding in HTA, including examples of successful programs overseas, is provided below.

HISTORY IN HTA

Australia **has never had a separate bridging fund for interim access** to health technologies and medicines.

However, other ad-hoc funding programs for COVID-19 vaccines and specific drugs have been used in the past.

OVERSEAS EXAMPLES

England has the **Cancer Drug Fund**, which provides funding for promising cancer drugs. This occurs via a managed access agreement while additional data is being collected to address uncertainty.

The Innovative Medicines Fund in England is a similar program for non-cancer medicines.

THE PROBLEM

Australian patients are waiting longer than comparable OECD countries for medicines.

Australian patients wait, on average, **466 days** from TGA approval to PBS reimbursement.[3]

A bridging fund could **help expedite access** (at TGA registration).

A BRIDGING FUND IS A MECHANISM TO PROVIDE PATIENTS FASTER ACCESS TO TRANSFORMATIVE MEDICINES AND TECHNOLOGIES.

BRIDGING FUND OPTIONS

The HTA Review Options Paper presented several options relating to the establishment of a bridging fund.

OPTIONS PRESENTED IN THE HTA REVIEW OPTIONS PAPER

Early identification and **nomination** via horizon scanning and/or designation on a Priority List of high unmet clinical need conditions.

Approach should provide the Committee with options to make recommendations for conditions, and recommendations that inform **further price and access negotiations**; or facilitates **finalisation of price** and **access negotiations** prior to presentation for consideration.

Eligibility requirements to lodge TGA and PBAC submissions (simultaneously) for the health technology within 6 months of receiving first international regulatory approval (i.e. FDA/EMA).

Requirement for **parallel TGA/HTA Committee submission lodgement** as part of a broader overall approach to support timely recommendations.

Administration that enables **clinical data to be collected and reviewed.**

A clear **process for reassessment** and **final decision-making** i.e. managed entry.

"WE ARE HERE BECAUSE THERE ARE SYSTEM IMPROVEMENTS THAT NEED TO TAKE PLACE. AUSTRALIAN PATIENTS WAIT MUCH LONGER ON AVERAGE TO GET ACCESS TO MEDICINES."

"WE NEED TO BALANCE EQUITY AND FASTER ACCESS. WE NEED TO CONSIDER HOW WE MAKE THIS A FAIR SYSTEM, AND HOW WE MAKE THE OVERALL SYSTEM FASTER TO IMPROVE THE STANDARD IN AUSTRALIA."

LEARNINGS FROM THE DISCUSSIONS

WHAT DO STAKEHOLDERS WANT FROM THIS?

This differs depending on the type of stakeholder: consumers, government, industry, patients, clinicians and academics.

It is generally agreed that there needs to be an element of transparency. All stakeholders involved need to understand how the system works. There needs to be agreement across stakeholders as to the purpose of the fund, and clear eligibility criteria.

WHAT ARE THE BARRIERS?

Some of the barriers identified include government support and the source of funding. Complex administration and legislation can also act as barriers.

WHAT ARE THE ENABLERS?

Some of the barriers identified can also act as enablers, such as legislation change, government support and funding.

When designing the fund, overseas and local examples should be leveraged. In doing so, we do not need to design the system from a blank slate, as there are learnings and adaptations that can be made from what already exists.

KEY THEMES:

TRANSPARENCY
EQUITY
FLEXIBILITY
PURPOSE

"TIME FRAMES AND GOVERNMENT PROCESS NEEDS TO BE FOLLOWED TO IMPROVE CERTAINTY."

"THE BENEFITS OF A BRIDGING FUND ARE GENERALLY AGREED, BUT THERE IS RISK IT MAY BECOME A BACKDOOR ENTRY TO A PBAC SUBMISSION."

"FUNDING COULD COME FROM COMPASSIONATE ACCESS PROGRAMS. THIS ENABLES A BRIDGE AND WOULD COVER ALL DRUGS. BUT REQUIRES OUTCOMES AND DATA COLLECTION TO BE IMPROVED."

- Does the funding need to come from a new 'pot' of money?
- Are we trying to fund medicines ahead of PBAC/cost effectiveness assessment?
- Should the fund be separate to the HTA process?
- If there needs to be a new source of funding, where does the pharmaceutical rebate go? What does it get used for?

LEARNINGS FROM THE DISCUSSIONS

KEY THEMES:

BROAD CONSULTATION
POLITICAL SUPPORT
FASTER ACCESS
CLARITY

"THIS TOPIC HAS THE MOST POTENTIAL TO MAKE A DIFFERENCE TO PATIENT ACCESS."

"WE NEED TO BE CLEAR ON WHAT WE ARE ASKING FOR AND ADAPT THE LANGUAGE TO GET MORE SUPPORT FROM STAKEHOLDERS. WE NEED TO DECIDE WHEN THIS WILL BE USED AND WHAT MEDICINES WOULD SIT IN THIS FUND."

WHAT IS NEEDED TO ACHIEVE SUCCESS?

To achieve success there needs to be clarity around the source and structure of the fund, as well as the timing of when it should apply (pre/post TGA). To design the fund, revisiting expert papers and drawing on international examples would improve the likelihood of success.

PRIORITISATION OF ACTIVITIES

There needs to be horizon scanning to identify emerging medicines, as well as a retrospective analysis of medicines that could have benefited from this fund in the past. The size of the fund and the specific eligibility criteria needs to be determined.

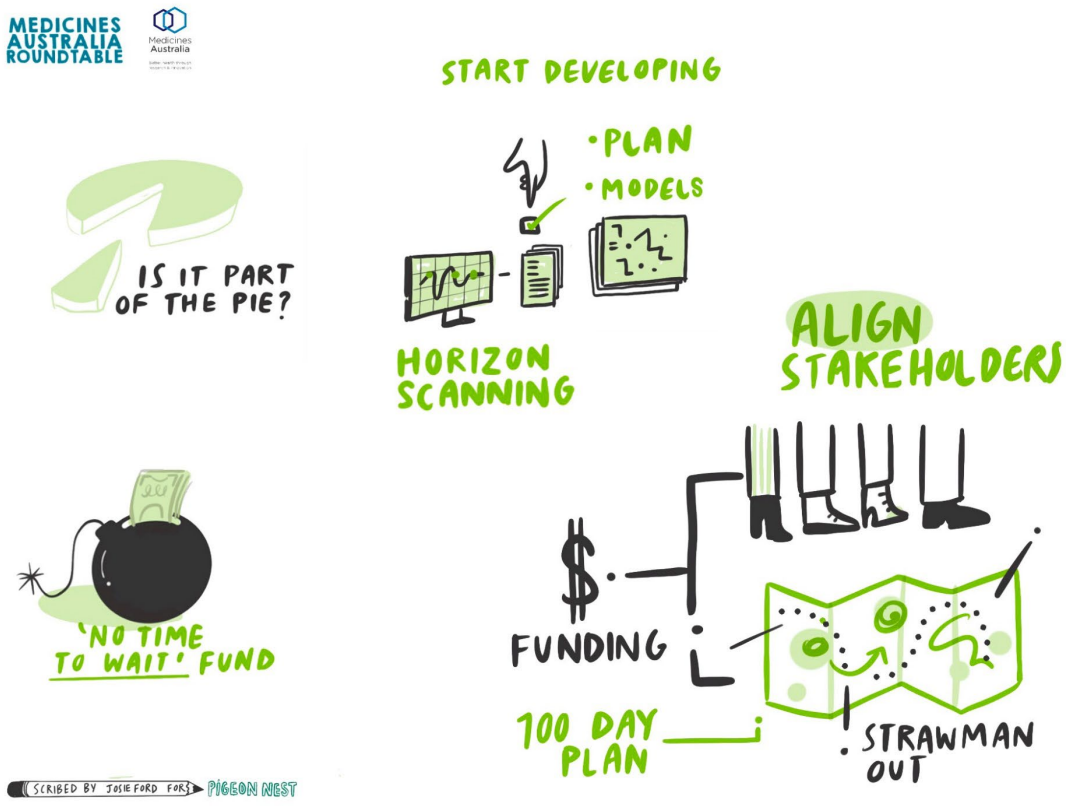
Developing this model would require broad consultation and the assembly of a cross-functional stakeholder group (including industry, academics, government, and the broader community).

ACTION PLAN

There needs to be alignment across all stakeholders and a formal call to action to government. This should be implemented as a priority when considering all the recommendations from the HTA Review.

- How do we manage ongoing subsidy?
- How do we frame this as an investment and not a cost?

BRIDGING FUNDING



INSIGHTS

The bridging fund has arguably the greatest potential to enhance patient access in Australia, making it a crucial topic for consideration and implementation. Following the release of the final report of the HTA review, it is important to maintain momentum in progressing the implementation of this fund. The success of this initiative will rely on government support and commitment to funding.

A critical first step is for all stakeholder groups to align on the fund's purpose. Through broad consultation, stakeholders must come together to develop a model that clearly defines the structure, timing, eligibility criteria, and funding sources.

MANAGED ENTRY

A CONDITIONAL ARRANGEMENT BETWEEN A MANUFACTURER AND PAYER THAT ENABLES EARLIER REIMBURSEMENT OF A HEALTH TECHNOLOGY

USE OF MANAGED ENTRY

Managed entry is a conditional arrangement between a manufacturer and payer to enable earlier reimbursement of a health technology. A brief background on managed entry in the context of Australian HTA is provided below.

HISTORY IN HTA

The Managed Entry Scheme was first **introduced in 2011** (Managed Access Program from 2015), where PBAC may recommend listing at a price justified by existing evidence, pending more conclusive evidence.

It is used in **areas of high unmet need**, where the PBAC would not otherwise recommend listing at the proposed price due to the level of uncertainty.

UTILISATION

Managed entry is **poorly utilised** in Australia.

This is due to agreements lacking flexibility and transparency, and there being **limited incentives** or benefits for the Sponsor.

PROGRESS TO DATE

Between 2011 and 2022, **4 oncology and 2 CF products** were listed via a MES/MAP.*

In December 2023, patisiran for **hATTR amyloidosis** was recommended for a MAP, with a 3-year reassessment required.[4]

In the last 3 years, the PBAC have recommended **MAPs for only 2 drugs**.**

*Ipilimumab (melanoma), crizotinib (NSCLC), trametinib with dabrafenib (melanoma), pembrolizumab (melanoma), ivacaftor based CF treatments x 2
**Trikafta and Onpattro

THE AIM IS TO ADDRESS THE UNCERTAINTIES SURROUNDING THE VALUE, UPTAKE AND PERFORMANCE OF EMERGING MEDICINES AND TECHNOLOGIES TO PROVIDE TIMELY PATIENT ACCESS TO THESE ADVANCED TREATMENTS

MANAGED ENTRY OPTIONS

The HTA Review Options Paper presented several options relating to the use of managed entry. These options reflect the evidence presented, and the input received from participants during the HTA Review consultations.

OPTIONS PRESENTED IN THE HTA REVIEW OPTIONS PAPER

Revised guidance and policy arrangements that **encourage the creative proposition and utilisation** of managed entry arrangement instruments by the respective parties.

Supported by **more explicit HTA committee recommendations** enabled by appropriate changes to current policy and legislation to facilitate greater uptake.

Support **continuous assessment of real-world data** and **timely access** to health technologies.

Provide **more options** to sponsors and the Commonwealth to **engage with uncertainty more constructively and collaboratively.**

LEARNINGS FROM THE DISCUSSIONS

WHAT DO STAKEHOLDERS WANT FROM THIS?

Stakeholders want a clear, transparent and explicit process, with an understanding of how managed entry fits within other aspects of the HTA process.

In developing these agreements, patients should be included from the beginning, acknowledging that the goal is to achieve faster patient access to innovative therapies.

There needs to be an element of trust from all stakeholders involved.

KEY THEMES:

RE-FRAMING
PATIENT-CENTRIC
FLEXIBILITY
TRANSPARENCY
CO-DESIGN

"THERE IS A NEED TO RESET THE RELATIONSHIP BEFORE STARTING THE PROCESS."

WHAT ARE THE BARRIERS?

The current process lacks timeliness and efficiency, as resubmissions are often required. The guidelines lack detail and clarity for patients, clinicians and Sponsors.

For the Sponsor, managed entry is associated with pricing risks. They are resource intensive to pursue and there are often barriers related to data availability and applicability. These agreements lack flexibility and a clear exit process, which act as disincentives for managed entry uptake.

"THE ROLE OF THE TGA IS CRITICAL IN THE EARLY ENGAGEMENT PROCESS."

WHAT ARE THE ENABLERS?

A clear and transparent framework needs to be co-designed through authentic partnerships with stakeholders. The framework should consider the broader system and have clearly defined and agreed parameters that balance flexibility and certainty.

Tools such as horizon scanning could be utilised to identify suitable products and enable early engagement. There needs to be appropriate expectations around data collection, and predictable and manageable risk around the future price impact. A clearly defined exit process needs to be developed.

- Are risk sharing arrangements considered an enabler?
- Would third party assessment of evidence be beneficial in this space?

LEARNINGS FROM THE DISCUSSIONS

KEY THEMES:

TRUST
CO-DESIGN
TRANSPARENCY
PATIENT CENTRIC

"WE NEED CO-DESIGN FROM THE FIRST CONVERSATION."

"REFRAMING MANAGED ENTRY AND REPLACING THE WORDING WITH SOMETHING LIKE "PROVISIONAL" WOULD BE HELPFUL FOR PATIENT UNDERSTANDING. IT IS DIFFICULT TO DESCRIBE WHAT 'MANAGED ENTRY' MEANS TO PATIENTS."

WHAT IS NEEDED TO ACHIEVE SUCCESS?

There needs to be a true partnership with all relevant stakeholders so they can collectively determine "what good looks like". This requires trust from all parties and a true co-design process, ensuring everyone is aligned on the goals and purpose.

Reframing the term "managed entry" as "provisional access" should be considered to help stakeholders align. The program needs a well-defined framework and a commitment to regular review, which could involve key performance indicators.

PRIORITISATION OF ACITIVITES

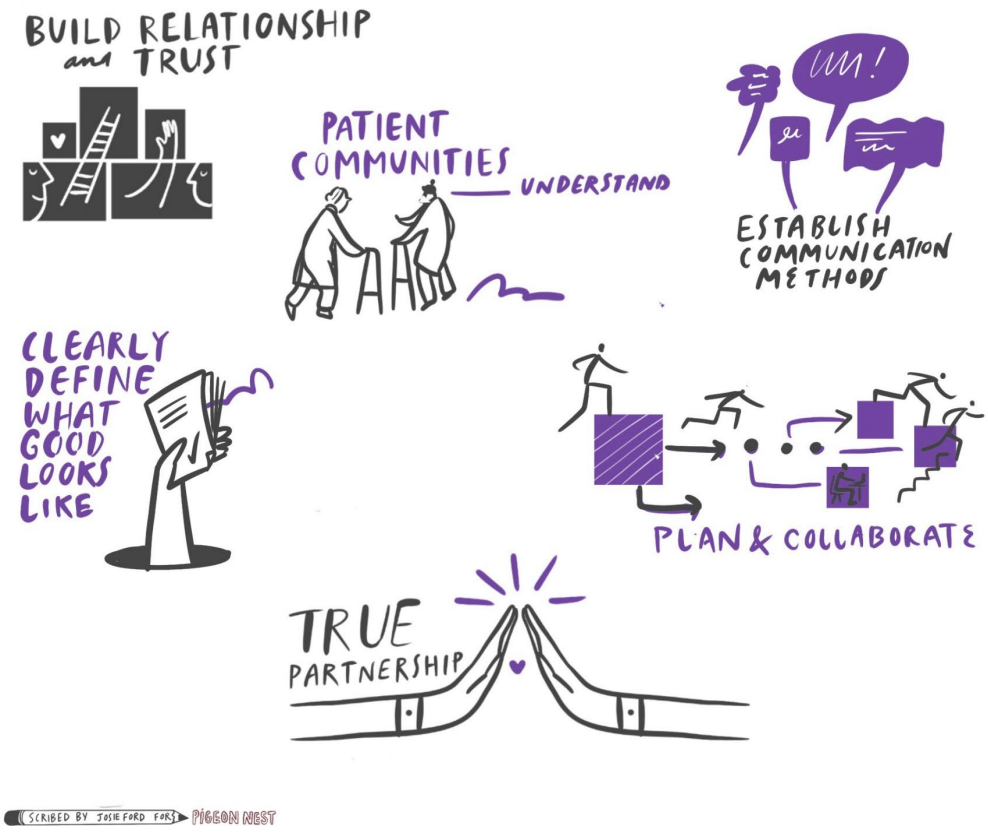
Relevant stakeholders need to be identified, and goals should be agreed upon in true co-design. There needs to be process maps and a framework developed, as well as an advocacy plan.

ACTION PLAN

Communication methods with stakeholders need to be established, and through co-design, a guide needs to be formulated. Guidance for data collection and resource requirements should be determined, as well as defining transparency and standardising plain language updates for the public.

- How do we get to a place of reform for managed entry that works for the patient?
- How do we develop clarity through a framework when every product has different issues?

USE OF MANAGED ENTRY



INSIGHTS

Managed entry is frequently cited by stakeholders as a key strategy for achieving faster access to health technologies. Despite widespread recognition of its potential, these agreements are often poorly understood and underutilised in HTA.

To address this, we must rebuild trust in this space, reset and reshape stakeholder relationships, and promote the adoption of managed entry. One approach could be to reframe "managed entry" as "provisional access," to better reflect its purpose.

Collaborative partnerships are essential for developing a new framework for managed entry. This framework should provide clear guidance on when managed entry is appropriate, enabling proactive proposals to prevent delays. Involving patients from the outset and throughout the process is crucial for ensuring true co-design and alignment with patient needs.

ATTENDEES

Medicines Australia

AUSTRALIAN PATIENT ADVOCACY ALLIANCE

AstraZeneca

rare cancers AUSTRALIA

Bristol Myers Squibb®

Neuroblastoma AUSTRALIA

Omico.

pvi

Pfizer

PATHOLOGY TECHNOLOGY AUSTRALIA

ucb

SAVE OUR SONS DUCHENNE FOUNDATION

AMGEN

abbvie

IDFA Immune Deficiencies Foundation Australia

HTANALYSTS

Roche

UNSW SYDNEY

Brain Foundation

BIOMARIN®

Confluence Health Consulting

DRAGON CLAW

Biogen

Arthritis AUSTRALIA

Australian Patients Association

NeuroEndocrine Cancer Australia

Dementia Australia®

sanofi

Hunter Medical Research Institute

PCPA PRIVATE CANCER PHYSICIANS OF AUSTRALIA

Eczema Support AUSTRALIA

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APPENDIX

VALUE FRAMEWORK BREAKOUT SESSION 1

WHAT WAS PRESENTED

WHAT DO STAKEHOLDERS WANT FROM THIS?

THE PATIENT PERSPECTIVE

- *A qualitative approach that is simple, effective, less academic, and does not divert resources.*
- *Transparency in how the patient's input into the system has had an impact on decision making. Currently we don't know how much these non-quantifiable aspects are impacting decision making. We don't just rely on the ICER to make decisions, but we don't know what else is considered. There needs to be a more equitable relationship. This should be a reflection of a meeting, not an extra step.*
- *The framework and elements need to be agile and flexible, as needs can vary according to the condition and patient group.*
- *QALYs do reflect improvements in QoL, but there is sometimes a disconnect between trial evidence and real-world populations.*
- *Inclusion of considerations specific to First Nations Peoples. E.g. What Matters 2 Adults wellbeing measure,[5] which is specific to Aboriginal and Torres Strait Islander Peoples and could be used instead of QALYs.*
- *Should reveal the critical questions and key drivers the committee was thinking about and how these came out.*
- *It should help committee members do their job effectively.*

THE ACADEMIC PERSPECTIVE

- *There may be a trade-off between perceived transparency and the fact that you want to get broader values into the process.*
- *The value of HTA takes us away from the loudest voices being supported. There is a risk that the value framework could move us away from clear HTA decision making.*

THE CLINICIAN PERSPECTIVE

- *QoL data are removed from the patients affected by the condition; the only people in a position to talk about value framework is the patient.*
- *Risk that there will be no community involvement if we have simultaneous regulation and reimbursement.*
- *Should capture secondary elements e.g. My parents have to look after my kids while I get treatment.*
- *Certainty that the evaluators and PBAC/MSAC take these into account, whether quantitative or qualitative.*
- *Need to ensure that a framework doesn't cause delays.*

WHAT ARE THE BARRIERS?

THE PATIENT PERSPECTIVE

- *Patient experience hasn't historically been as important as it is now.*
- *Imposing a new process rather than trying to integrate into existing systems to avoid overcomplication.*
- *The National Medicines Policy (NMP) was less explicit about equity than it is now – new NMP focusses on specific populations e.g. First Nations.*

THE ACADEMIC PERSPECTIVE

- *There are methodological barriers, including the tools to implement.*
- *Resistance to less quantifiable aspects impacting on objective decision-making; hard to use without quantifying.*
- *Timeliness of including patients in co-design processes. Need to balance time to take into account patient perspective with the need to get treatments to patients.*
- *Will it make a difference if we take patient input into account? Will it shift decisions? All conditions have patients. BUT there is a community imperative to consider patient perspectives.*

THE CLINICIAN PERSPECTIVE

- *Uncertainty about how a qualitative framework including things like productivity will impact ICERs.*
- *Difficulty in applying value frameworks in a quantitative way to ensure equity and diversity.*
- *Even if the inclusion of patient/ community perspectives makes no difference to the outcomes, they should be considered. Currently it feels like these aspects are not important, but they are.*

OTHER BARRIERS

- *First mover barrier – want to see someone else succeed first.*
- *Easy to 'hide behind' a number e.g. QALYs; qualitative/human elements are more difficult to 'hide behind'.*

WHAT ARE THE ENABLERS

THE PATIENT PERSPECTIVE

- *Elevation of the patient voice.*

THE ACADEMIC PERSPECTIVE

- *First stage, specifying what's important; second stage, applying these prespecified outcomes to apply rigor.*

THE INDUSTRY PERSPECTIVE

- *Bucketing values similar to Value Fountain presented in Value of Pathology Technology Report.*[6]

THE CLINICIAN PERSPECTIVE

- *Coming together as a whole community with all stakeholders to elevate the collective voice.*

THE INDUSTRY PERSPECTIVE

- *Values framework needs to reflect what Australians expect from health and access. They need to be an explicit measurement of Australian values.*
- *Development of a framework that allows incorporation of values, whether quantitative or qualitative.*
- *Clearly defined place in the process: beginning? End? Middle? Relative to the economic model?*

OTHER ENABLERS

- *Written into the Options paper with a proposed process for inclusion.*
- *Wealth of literature.*
- *COVID-19 example – what did we do right? How can we learn from this?*

VALUE FRAMEWORK BREAKOUT SESSION 2

WHAT WAS PRESENTED

WHAT IS NEEDED TO ACHIEVE SUCCESS?

- *Simplicity.*
- *Getting everyone on the same page.*
- *Defining the problem.*
- *Defining the purpose of the framework.*
- *Defining the elements to be measured: figuring out the data points.*
- *Analyse medicines that didn't get through the system to understand why.*

PRIORITISATION OF ACTIVITIES

- *Cross-healthcare system workshop and consultation process to outline elements of value and how they will be used in an appraisal.*
- *Develop a list of values based on those in PBAC Guidelines and a literature review to identify what's missing.*
- *Validate the list through co-design with the community to prioritise those values.*
- *Define how they will be measured.*
- *Define how the PBAC will use the values.*
- *PBAC to report how the values are used in deliberations.*

ACTION PLAN

- *Advocate for a broad co-design process.*
- *Develop a multi-stakeholder proposal.*

ADDITIONAL DISCUSSION

DEFINE THE PROBLEM

- *Understand what the community values in HTA.*
- *Some HUCN medicines aren't making it through the system - did they get rejected for the right reasons?*

WHAT IS THE PURPOSE OF THE FRAMEWORK?

- *What's not in the clinical data and how do you rank these elements? How wide are we allowing the framework to extend?*
- *Tool for the PBAC/MSAC to use.*
- *Transparency for community about what was considered and how it was used.*
- *Reflect what's important to the community, especially priority groups.*

SOLUTIONS

- *Ask the community to define and prioritise what they value.*
- *Simple tool that can be implemented now.*
- *Measurable framework - we need to establish rigor around the framework.*
- *Modifiers: Japan, UK; loading: First Nations Peoples, end-of-life care.*
- *Have patient voice upfront e.g. through early PICO consultation.*
- *Review previous submissions and look at what's been considered by PBAC outside of the economic model.*

OTHER THOUGHTS

- *Utilities used in HTA don't reflect the experience of patients with that condition.*
- *Need to start with patients. This is not a shared language, often patient groups need to do a translation for patients, resulting in time and knowledge lost.*
- *Need to ensure that the input we get from people isn't affected by their own bias e.g. Self-reported health in some First Nations communities are high despite being in poor health.*
- *Need principles underpinning the framework, such as inclusivity.*

ADAPT LIST OF VALUE ELEMENTS TO INCLUDE SECONDARY BENEFITS / ADDITIONAL ELEMENTS OF VALUE

- *Co-design processes.*
- *Weight and authority. Broad reach. Workshop that includes all relevant players.*
- *Run a consultation process. Process where anyone that wants to build it can i.e. broader community.*
- *Leverage literature e.g. What matters 2 Adults[4] – Aboriginal and Torres Strait Islander community.*
- *Principles of how value elements should be used.*

VALIDATE VALUE ELEMENTS

- *Survey the community (communities) on how they value secondary benefits & prioritise.*
- *Need to recognise complexity. Difficulty of surveys.*
- *PBAC & DoH.*
- *Literature.*
- *Importance of dialogue.*

HOW DOES IT ACTUALLY WORK IN AN APPRAISAL?

- *Explicitly outline how framework is used in an appraisal.*
- *Articulate how it is transparently reported in a consideration.*
- *Is a modifier what we need in Australia?*
- *Early agreement of the value elements, i.e. when the PICO is agreed.*

GUIDANCE ON METHODOLOGY

- *Articulated new methodologies for value elements.*

WHAT IS THE BURNING PLATFORM WE ARE TRYING TO SOLVE?

- *Academic examination of medicines that were not approved to understand 'why' from a value perspective.*
- *Ensure the PBAC articulate how value elements are considered and how they impact decision making.*

BRIDGING FUND BREAKOUT SESSION 1

WHAT WAS PRESENTED

WHAT DO STAKEHOLDERS WANT FROM THIS?

- *First need to define stakeholders, as different stakeholders may want different things. There is the consumer perspective, government (Department, Ministry, MP's), industry, broader patient community (can include people with lived experience and wider community), clinicians, and the academic community.*
- *Need to consider the purpose of the bridging fund. Is it about faster access? Universal access? Equitable access? Sustainable access?*
- *There needs to be an element of transparency. e.g. neuroblastoma funding example, there needs to be transparency around negotiations and the process/pathway.*
- *Size of the fund? What would be acceptable to the government? There are questions around this. We need to make sure money isn't being taken from somewhere else. It has to be equitable. Are we incorporating the LSDP?*

WHAT ARE THE BARRIERS?

- *Parts of government don't necessarily want faster access.*
- *The following risks were identified: that the fund may be too niche, having a cap, equity, and the risk it could become an LSDP bridge? How do we avoid this becoming just a bureaucratic process?*
- *Source of funding (state vs federal).*
- *Needs to be flexible, but too much flexibility can reduce predictability.*
- *Not having robust horizon scanning in place.*
- *Setting of administration.*
- *Legislation change.*

WHAT ARE THE ENABLERS?

- *Horizon scanning to identify innovative therapies.*
- *Flexibility.*
- *Develop transparent criteria and a framework that provides predictability for sponsors, patients and government.*
- *Valuable system change, and an iterative process involving regular review.*
- *Real world data and an appropriate framework to capture it.*
- *Precedence: Utilise overseas examples such as the Cancer Drug Fund and Innovative Medicines Fund in the UK, as well as local examples such as the COVID-19 pandemic and neuroblastoma funding.*

BRIDGING FUND BREAKOUT SESSION 2

WHAT WAS PRESENTED

WHAT IS NEEDED TO ACHIEVE SUCCESS?

- *There needs to be alignment on where the money comes from and how the fund is structured.*
- *Bridging fund needs to 'grow the pie'. We don't want it to be taking funding from somewhere else.*
- *The fund should be uncapped, but there needs to be tight inclusion criteria.*
- *There needs to be clarity around the timepoint at which bridging funding should apply. Is it pre or post TGA?*

- *Leverage expert papers and look to other examples of how this has been done (e.g. Cancer Drugs Fund, Innovative Medicines Fund).*

PRIORITISATION OF ACTIVITIES

- *Implement horizon scanning to identify innovative and emerging medicines and technologies.*
- *Undertake a retrospective assessment of products that could have gone through a bridging fund.*
- *Determine the budget for the fund.*
- *Develop specific eligibility criteria and categorise products e.g. high unmet clinical need, live saving etc.*
- *Develop a stakeholder group, design a plan and develop options for implementation. Undergo consultation. This could be modelled similar to the National Reconstruction fund.*

ACTION PLAN

- *Decide as an industry to formally call on government for a bridging fund.*
- *Get alignment across all stakeholders .*
- *Straw person for consultation, implement a 100-day plan.*
- *Time frame: we want an election commitment, with implementation in the first year of government.*

MANAGED ENTRY BREAKOUT SESSION 1

WHAT WAS PRESENTED

WHAT DO STAKEHOLDERS WANT FROM THIS?

- *A clear and explicit process.*
- *Understanding of how managed entry fits within other aspects of the HTA process (i.e. bridging fund, LSDP).*
- *Including patients from the beginning of the process. Data sovereignty, access. Understanding of what matters to the patient.*
- *Clear understanding of what data needs to be collected. LTFU studies versus RWE.*
- *Complete transparency about the process for all stakeholders.*
- *Industry should not be solely responsible.*
- *Trust.*

WHAT ARE THE BARRIERS?

- *Feels biased – perceived as just a mechanism to drive price down.*
- *PBAC is the gatekeeper to recommending managed entry.*
- *Data availability and applicability.*
- *Expensive to locally collect data.*
- *Resource requirements from DoHAC is burdensome, less likely to adopt.*
- *Adversarial and combative between key stakeholders.*
- *Immature vs uncertain data.*

WHAT ARE THE ENABLERS?

- *Start from scratch.*
- *Third-party assessment of evidence.*

- *Accepting reasonable inherent uncertainties in the evidence.*
- *Considering the global context.*
- *Reframing – consider what is this for (early access for patients).*
- *Horizon scanning to identify appropriate products.*
- *Authentic partnerships between stakeholders to collaborate and come to mutually beneficial outcome. Co-design.*
- *Providing clarity on when managed entry is and isn't suitable.*
- *Clear exit.*

MANAGED ENTRY BREAKOUT SESSION 2

WHAT WAS PRESENTED

WHAT IS NEEDED TO ACHIEVE SUCCESS?

- *Clearly define what good looks like for all stakeholders.*
- *True partnership for all relevant stakeholders, each with clearly defined roles. Including the building of trust.*
- *An informed patient community.*
- *Early and productive engagement. Companionship with the TGA.*
- *Early identification of when managed access is appropriate.*
- *Updated framework for review of managed access. KPIs.*
- *Commitment from PBAC and DoHAC for consideration.*
- *A well-defined, clear framework that is affordable and feasible. Define who is collecting the data.*
- *Clarity around what happens when you succeed, and what happens when you don't?*

PRIORITISATION OF ACTIVITIES

- *Identify stakeholders.*
- *Agree on goals amongst all stakeholders.*
- *Joint leadership. True codesign.*
- *Clear mechanism for communication.*
- *Process maps, framework development.*
- *Rebranding – suggest using the word 'provisional'?*
- *Advocacy plan.*

ACTION PLAN

- *Establish communication method with stakeholders.*
- *Co-design from the first conversation.*
- *Formulate guide for scenarios where the sponsor will be required to collect more data.*
- *Feasibility of codification of local data collection should not be assumed.*
- *Determine cost of collection.*
- *Standardisation of status, clear plain language publication of updates for the public.*
- *Defining transparency regarding data sharing.*

ONLINE FORUMS AND 1 ON 1 INTERVIEWS

WHAT WAS DISCUSSED

VALUE FRAMEWORK

WHAT DO STAKEHOLDERS WANT FROM THIS?

- *The things that are important to patients need to be included in the value framework. It needs to be a patient-centric approach.*
- *There needs to be flexibility in what is included, as different patient groups have different needs.*
- *The value of knowing needs to be included as a consideration. This is particularly important for patients with rare diseases.*
- *Transparency to understand how decisions are made.*
- *Co-design with consumers.*
- *Mutual trust and understanding of definitions.*
- *Equity considerations.*

WHAT ARE THE BARRIERS?

- *One size does not fit all, there are specific challenges unique to each disease (and specific to rare diseases).*
- *Access to data*

WHAT ARE THE ENABLERS?

- *Broad co-design.*
- *Building a flexible framework.*
- *Pre-submission meetings to identify value elements specific to the therapy early on in the process, as to not delay patient access.*
- *Categories similar to the US ICER framework could be used. These need to be flexible and agreed upon.*
- *Incorporating the patient voice.*
- *Culture change.*

WHAT IS NEEDED TO ACHIEVE SUCCESS?

- *Ways to ensure patients are involved from the beginning, even as early as the trial design.*
- *Values need to be collected from the start and part of trials.*
- *How we measure success needs to be determined, we need performance indicators.*
- *Need Sponsors to have an appetite for putting medicines for rare diseases through the reimbursement process, currently they aren't a priority for because of the small patient numbers.*
- *Patient and clinician engagement.*

OTHER COMMENTS

- *There needs to be consideration of indirect health outcomes such as long term or future cost savings due to things like disability/NDIS payments avoided.*
- *An iterative process for the development of the value framework is appropriate.*
- *There needs to be measures on how this reform is doing. There needs to be a regular review process to ensure it is fit for purpose*
- *Early advice, which could be given during pre-submission meetings or otherwise, would provide an opportunity for all involved to identify areas of uncertainty and understand where the gaps are from the start.*
- *We need to value medicines more; this may mean accepting a higher price.*
- *There needs to be a weighting of value elements in the framework. In some circumstances QoL outcomes should have more weighting than other outcomes. However, there is difficulty in getting the balance right.*

- *How do we speed this up while making it a consultative process?*
- *We need to define success to be able to measure the success of the framework. Does success mean equity? And how do we measure and define equity?*

BRIDGING FUND

WHAT DO STAKEHOLDERS WANT FROM THIS?

- *An ability for non-commercial Sponsors to get medicines through (important for rare disease therapies where there is limited incentive for Sponsors).*
- *The fund to coexist with existing mechanisms and criteria to select HUCN patients and identify which treatments would be beneficial. This could link to expert clinical panels.*
- *Bridging fund criteria needs to align with the PBS restriction so patients can have continued access.*
- *Clear agreements on funding i.e. how long for? What does off ramping look like? Will eligibility criteria change between interim funding and PBS listing?*
- *Access to treatments that are available overseas.*
- *Needs to be broad.*

WHAT ARE THE BARRIERS?

- *There are many complexities to consider with implementing a bridging fund.*
- *Getting legislative change.*
- *Adequate resourcing.*

WHAT ARE THE ENABLERS?

- *Horizon scanning to identify what therapies are coming, and to identify areas of HUCN.*
- *Clearly defining the principles and criteria e.g. HUCN.*
- *Real world evidence.*
- *Consideration of the patient perspective.*

WHAT IS NEEDED TO ACHIEVE SUCCESS?

- *Raise the patient voice. Communicate how many lives can be saved if a bridging fund is implemented. e.g. for rare diseases, a bridging fund is necessary because of the lack of options available.*
- *Clear and transparent eligibility criteria.*
- *Political opportunity.*
- *Apply an equity lens.*

OTHER COMMENTS

- *A fund could be beneficial for treatments that are on the PBS for another indication but not available for rare diseases*
- *Repurposing medicines is important, it needs to be easier to get a PBS listing for multiple indications.*
- *The Australian market is often too small for companies to pursue reimbursement – the fund needs to address this issue.*
- *Regarding the LSDP and how it fits with this topic – either it all flows together or the LSDP needs to be reformed so that you don't need to fail the PBAC submission pathway first.*
- *There is an interaction between bridging funding and managed entry. Managed entry could be used with an interim funding arrangement. The managed entry agreement would be used to earlier patient access.*
- *Patient community groups want trial access, and to be involved in the global design of trials. There is often limited opportunity for Australian patients to be a part of trials managed overseas.*
- *A bridging fund could work as an incentive for Companies to seek reimbursement in Australia.*

QUESTIONS

- *How does the price get agreed on? Is there a mechanism to determine this?*
- *The aim of the fund is to provide patient access in a reasonable time frame, but what do we deem to be a "reasonable timeframe?"*
- *Will a bridging fund discourage Sponsors from setting up Compassionate Access Programs?*
- *How would this interact with the medical treatment overseas program?*

USE OF MANAGED ENTRY

WHAT DO STAKEHOLDERS WANT FROM THIS?

- *Using real world evidence as supportive evidence to address uncertainty.*
- *Clinical consensus as a pathway to earlier access.*
- *A clear framework to provide certainty as to when managed entry is needed.*

WHAT ARE THE BARRIERS?

- *Lack of available data.*
- *Complexity in the current system*

WHAT ARE THE ENABLERS?

- *A transparent framework*
- *Incentives for Sponsors.*
- *Leveraging the existing structures.*

WHAT IS NEEDED TO ACHIEVE SUCCESS?

- *Setting up the right environment and incentives for Sponsors.*
- *More transparency and equity.*
- *Trust across all stakeholders.*
- *A true sharing of risk.*
- *Advocacy.*

OTHER COMMENTS

- *Real world evidence plays a big part in this and needs to be tied to the value framework.*
- *There needs to be equitable access for Australian patients. Drugs available overseas that have FDA or European Medicines Agency approval should be available here.*

THE HTA REVIEW FINAL REPORT

MEDICINES AUSTRALIA SUMMARY

REVIEW RECOMMENDATIONS

The Review proposes 50 reform recommendations, which, when implemented as a complete package, will accelerate patient access to new medicines and vaccines in Australia.

Grouped in themes, these recommendations reflect consistent areas of concern and opportunity highlighted by stakeholders throughout the Review process.

The reforms recommended by this Review provide stakeholders and decision-makers with the tools and processes to:

1. Address inequities in access;
2. Improve timely access to medicines;
3. Improve engagement; and
4. Invest in HTA capability to make it adaptable and future proof.

Clear need for urgent system reform

The Review clearly states the urgent need for system reform, recognising the profound impact and negative consequences for individual patients caused by delays in accessing new medicines.

Some of the failings of Australia's current HTA process identified through the review process include:

- Health technologies are not funded in the shortest time possible.
- Delays are because the economic evaluation is being used as a proxy for negotiation of the prices paid.
- HTA pathways in Australia are more complex than they need to be.

Intended outcomes following implementation of recommendations

The Review proposed that with implementation of the proposed reforms:

- The timeframe for Australians to have subsidised access to these high added therapeutic value therapies would be around 16 months faster (p65 HTA Review Report);
- By improving the alignment between the PBAC and ATAGI pathways, processes, and secretariat functions, and utilising the same evaluator group for both, the time to HTA recommendation for vaccines will be reduced by 18-22 weeks, or around 40% of the current time (p69 HTA Review Report).
- The time for listing on the LSDP will be reduced by around four months, through removing the additional steps in this pathway, and providing additional clarity and certainty to sponsors (p81 HTA Review Report).

SUMMARY OF RECOMMENDATIONS

Addressing inequalities – First Nations peoples and children (Recommendations 1 & 2)

The Review brings into focus the challenges that First Nations peoples face in equitable access and the critical need to provide more equitable access to medicines. Recommendations include establishment of a First Nations Advisory Committee to advise PBAC and MSAC on priority populations indications for First Nations people with high unmet clinical need, First Nations representation on PBAC and a requirement for sponsor submissions to include consideration of the impact on health outcomes for First Nations peoples.

To improve paediatric patient access to medicines, the Review recommends the adoption of an approach for new listings on the PBS that is agnostic of age unless there are special circumstances that necessitate restricting access. The Review also recommends establishment of a working party to develop guidance on extending the use of TGA-registered therapies to paediatric populations and industry looks forward to working with stakeholders to realise the ambition of this recommendation.

System reforms to speed up patient access and recognise the value to patients

The Review recommends mechanisms to improve equitable and timely access across the entire HTA ecosystem, including for:

- New vaccines on the National Immunisation Program (NIP) (Recommendation 11);
- Life-saving drugs for people with ultra-rare diseases (Recommendation 14);
- Highly specialised therapies delivered through the Addendum to the National Health Reform Agreement (NHRA) [7] (Recommendation 13); and
- Therapies proposed for listing on the PBS and those with co-dependent technologies (p15 HTA Review Report).

Important system reforms identified in the Recommendations include:

- A cost minimisation submission differentiation pathway (Recommendation 41);
- A Bridging Fund targeting areas of high clinical need (Recommendation 20);
- A co-designed values framework (Recommendation 26); and
- Horizon scanning (Recommendation 47).

Important pathway reforms proposed as Recommendations 3-11 include:

- A series of overarching recommendations for all HTA funding and assessment pathways such as a "single front door", core HTA committees and triaging;
- A streamlined pathway for submissions using cost-minimisation analysis;
- An enhanced early resolution pathway;
- Case management; and
- Decoupling the TGA Delegate's overview from the PBAC advice.

Together these reforms will:

- Reduce the time and effort sponsors, the Department, evaluators and the PBAC spend on low-risk, simple submissions for therapies with no additional therapeutic advantage over existing alternatives;
- Ensure the funding and assessment mechanisms and levels are proportional to the complexity, risk, and potential benefit related to the submission;
- Reduce the time to access for therapies with high added therapeutic value.

Improved engagement (Recommendation 22-26)

Effective stakeholder engagement and transparency in decision-making is fundamental to achieving the goals of the National Medicines Policy and the reforms in this Review.

Notable recommendations to improve stakeholder engagement include:

- Recommendation 25 aimed at improving the involvement of consumers in HTA.
- Recommendation 23 addressing improvements to the HTA webpage to make it more user friendly.

- Recommendation 22 which involves publishing plain language summaries of PBAC submissions and more transparent description of the PBAC's deliberations.
- Recommendation 26 providing for the development of an explicit values framework.

The Enhanced Consumer Engagement Report, developed in parallel with the HTA Review, and co-designed with patient organisations and industry, is welcomed and is intended to improve patient engagement with the system.

Movements in the right direction

There is some movement in the right direction on issues related to timeliness of access to medicines; however, with some more boldness in thinking, could be strengthened for greater patient benefit.

Key performance indicators **(Recommendation 15)**

The Review commits to jointly owned performance targets, recognises the need for continuous review and improvement of the HTA system and notes the system cannot be left alone again for 30 years only to have another major Review. It recommends that greater than 90% of listings occur within 6 months of registration for cost-effectiveness submissions under parallel processing. Industry will continue to advocate for patient access within 60 days of registration as this is an achievable target.

Comparator selection **(Recommendation 40)**

Comparator selection issues must be dealt with by the Government for Australians to have confidence in access to the medicine on the PBS that is best for them. The Review acknowledges the problems and that the PBAC needs more flexibility when selecting the comparator.

It recommends an update to the PBAC Guidelines to provide flexibility. This will need to be tried and tested; however, it is unlikely to make a difference when the Department expects the legislation to be upheld. Industry will pursue legislative change.

Discount rate **(Recommendation 39)**

The Review recommends that the base rate discount rate for health technologies that have upfront costs and benefits that are claimed to accrue over a long period of time (such as gene therapies and some vaccines) be reduced to no lower than 3.5 per cent. The is a step in the right direction but does not meet the access needs of all Australian patients. Industry will continue to a further reduction in the discount rate.