Bringing Patient Centricity to Life

Exploring system reform opportunities that will enhance patient centricity in Australian healthcare

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Acknowledgements

Biointelect and Bristol Myers Squibb wish to acknowledge the ongoing support and participation of the following organisations and individuals who have contributed to this work, from the Roundtable to the development of this paper.

Health Consumer Organisations

Arthritis Australia

ausEE Inc - Support Network for Eosinophilic Disorders

Australian Patient Advocacy Alliance

Australian Patients Association

Can Assist

Canteen

Crohn's & Colitis Australia

CreakyJoints Australia

Head and Neck Cancer Australia

Heart Foundation

Hearts4Heart

Leukemia Foundation

Lung Foundation Australia

Lymphoma Australia

Melanoma Patients Australia (MPA)

Melanoma & Skin Cancer Advocacy Network (MSCAN)

MS Australia

Myeloma Australia

Neurological Alliance Australia

Ovarian Cancer Australia

Pancare Foundation

Patient Voice Initiative
Ann Single (HTAi, Chair of the Patient Citizen Involvement Group)

Psoriasis Australia

Rare Cancers Australia

The National Aboriginal Community Controlled Health Organisation

Government and Industry Representatives

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Terminology

Term	Definition
AUSPAR	Australian Public Assessment Reports
ARTG	Australian Register of Therapeutic Goods
BMS	Bristol Myers Squibb
CEEU	Consumer Evidence and Engagement Unit
DoHA	Department of Health and Aged Care
EAP	Early Access Program
HCO	Health Consumer Organisation
HCP	Health Care Professional
HTA	Health Technology Assessment
HTACCC	HTA Consumer Consultative Committee
HTAi	Health Technology Assessment International
ISPOR	International Society for Pharmacoeconomics and Outcomes Research
LSDP	Life Saving Drugs Program
MAP	Managed Access Program
MES	Managed Entry Scheme
MSAC	Medical Services Advisory Committee
MSW	Medicines Status Website
NIP	National Immunisation Program
NMP	National Medicines Policy
OECD	Organisation for Economic Co-operation and Development
Patient or Consumer	Used interchangeably in reference to individuals who utilise the health system to access care and therapeutic goods.
PBAC	Pharmaceutical Benefits Advisory Committee
PBS	Pharmaceutical Benefits Schedule
PSD	Public Summary Document
QALY	Quality Adjusted Life Year
QoL	Quality of Life
RCT	Randomised Controlled Trials
RWE	Real World Evidence
SIP	Summary of Information for Patients
TGA	Therapeutic Goods Administration
UK	United Kingdom

Executive Summary

The current environment presents many opportunities for a variety of stakeholders, including patients, to engage with the Australian health system and government on reform.

- Australians have never been more aware of the importance of efficient approval processes for medicines and vaccines following the COVID-19 pandemic;
- Recommendations from the 2021 Parliamentary Inquiry into the approval processes for new drugs and novel medical technologies[1] are expected to be considered by the Australian government in parallel with recommendations arising from the 2023 review of Health Technology Assessment (HTA) policy, processes and methods;[2] and
- The 2022 strategic agreement between the Commonwealth and the medicines industry includes a commitment to deliver enhanced consumer engagement with respect to applications to list new medicines on the Pharmaceutical Benefits Schedule (PBS).

All of these activities are occurring under the umbrella of a revised National Medicines Policy[3] (NMP) that is built upon the aim of achieving well-coordinated person-centred care.

Undeniably, we are on the precipice of significant healthcare system reform and through this process **we can bring patient centricity to life.** Patients are more informed than ever and are increasingly empowered to understand and manage their healthcare needs. While improving clinical outcomes for patients is a core goal of the Australian healthcare system, other stated objectives such as patient-relevant outcomes and patient experience[4], are challenging to capture and achieve in practice.

Effectively harnessing the patient voice in our healthcare decision making represents an opportunity to not only align with the principles of patient centricity but to shape the health system into one that delivers better access, equity, responsiveness, and efficiency.

One significant area within the Australian healthcare system is HTA, the methodology and processes employed to evaluate medicines. While the use of HTA can be broad, from early aspects of the medicines lifecycle, such as horizon scanning, through to registration and reimbursement, as well as later stage aspects including post marketing and real world data generation, we are most familiar with the use of HTA in Australia in determining which medicines are listed on the PBS.

The Pharmaceutical Benefits Advisory Committee (PBAC) an independent expert body appointed by the Australian Government, uses HTA to assess the clinical-effectiveness, safety and cost-effectiveness of medicines compared to other therapies. The PBAC is established under the *National Health Act*

1953 (NHA), with its primary role being to recommend to the Minister for Health which medicines should be subsidised under the PBS.[5] It is important to note that the PBAC are bound by the policy and legislation under the NHA.

Across the continuum of HTA, including the determination of medicines to be made available on the PBS, patient engagement is crucial to support decision making that prioritises Australian patients and their healthcare needs.

Effective and sustainable reforms to Australia's HTA processes must begin with the patient at the centre.

Important advances have been achieved in involving consumers in HTA specific to the reimbursement of medicines, including in Australia, although stakeholders acknowledge (and expect) that there are opportunities to i) further enhance the participation and contributions of Health Consumer Organisations (HCOs) and consumers in the reimbursement process and ii) broaden HCO and consumer involvement in HTA across the lifecycle of a medicine.

Bristol Myers Squibb Australia (BMS), supported by Biointelect, convened the November 2022 *Shaping Healthcare Together* Roundtable and a series of subsequent focus groups with over 20 representatives of HCOs in March - April 2023, to discuss and gather perspectives regarding Australia's HTA processes and how they can better align with the principles of patient centricity. BMS has worked with HCOs towards achieving common goals in system reform over the last five years, through the Broadening the Evidence project and is committed to initiatives that support the role of consumers and patients in healthcare decision making and health policy.

This white paper highlights the key concerns and aspirations of participating HCOs for reforming Australia's HTA system to meet the needs of Australian patients.

It makes recommendations regarding broad scale system reform, but also smaller, practical steps towards practice and process improvements. Recommendations range across the medicines lifecycle and aim to empower consumers and enhance their involvement in design, approval, access, and post marketing review of medicines. A partnership approach across Government, HCOs and Industry was seen as key to prioritising, shaping and implementing these recommendations.

Collating the learnings and information gathered during the focus groups with HCOs and discussions with key government and industry representatives, the insights were distilled into three central themes:

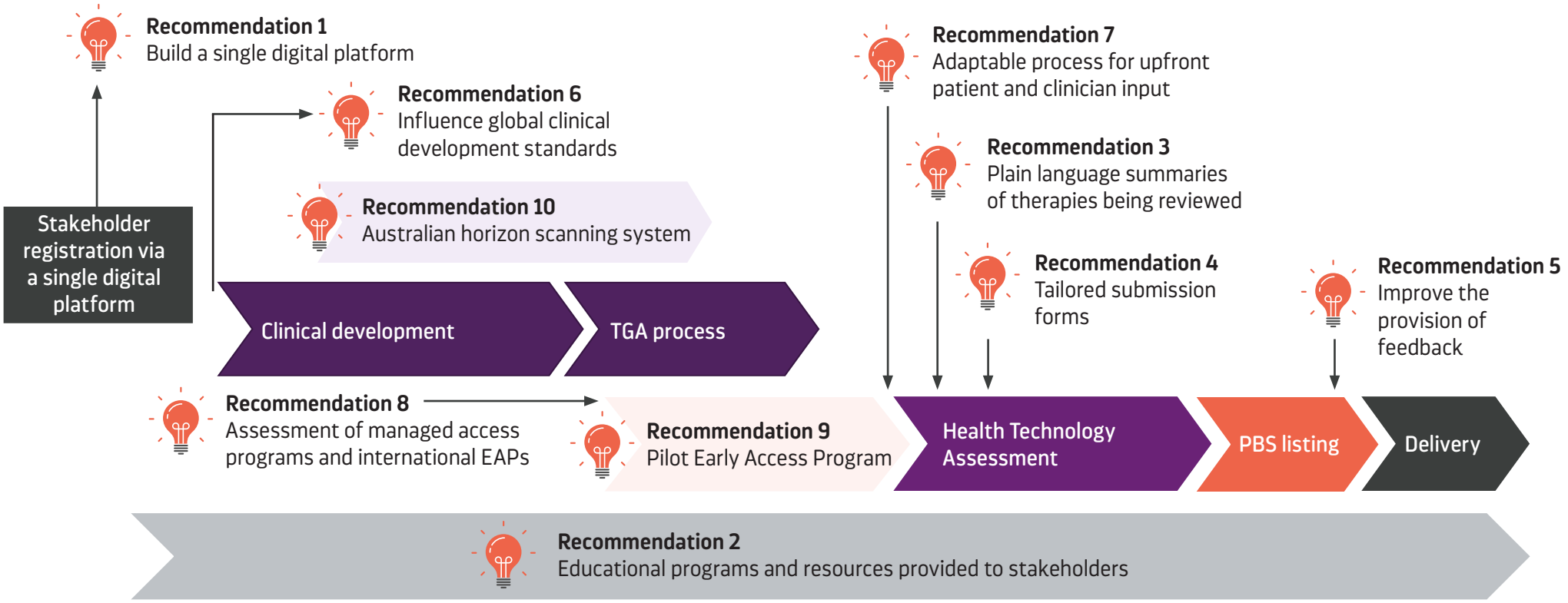
1. **Effective and Meaningful Consumer Engagement**
2. **Broader Value Considerations**
3. **Speed of Access to Medicines**

Across the three themes, ten opportunities and actionable recommendations for patient-centric system reform are explored. These are summarised in Figure 1 and are detailed in the following section.

Bringing Patient Centricity to Life

The proposed recommendations for reform are highlighted across the lifecycle of a medicine.

Figure 1: Summary of recommendations for improved patient input across the lifecycle of a medicine



Key Findings and Detailed Recommendations

Patient centric system reform refers to the transformation of healthcare and medicines delivery systems to prioritise the needs, preferences, and outcomes of patients. Opportunities and actionable recommendations for patient-centric system reform across the three central themes of this paper are outlined below.

Effective and Meaningful Consumer Engagement

It is widely accepted that patient voices are integral to the Health Technology Assessment (HTA) decision making process. Patients can provide insights based on the first-hand experience of living with their health condition and managing their healthcare. It was agreed within the focus groups that enhancements to existing processes should be considered to ensure that patients and their representatives are proactively informed, engaged and empowered to contribute to the processes that make medicines accessible and affordable to all Australians.

Interestingly, there was also discussion and support for greater transparency about a medicines' pathway from clinical trial development to registration, reimbursement and post marketing, noting that significant investment and resources would be required to establish and maintain a fit-for-purpose information platform across the spectrum of a medicines lifecycle.

Recommendation 1: Build a single digital platform or front door to access information about TGA and PBAC application status

To enable HCOs to access consistent, reliable, and up to date information efficiently, a single digital platform or front door would act as a single source of truth. It would include:

- Overview of the progress of a new therapy, from horizon scanning (while still in clinical development), through Therapeutic Goods Administration (TGA) intent to apply and application status;
- Pharmaceutical Benefits Advisory Committee (PBAC) submission status;
- Required future steps to Pharmaceutical Benefits Scheme (PBS) listing; and
- PBS listing and post marketing activities.

Details of the platform should be further explored; however, participating Health Consumer Organisations (HCOs) notionally agreed that this might be presented as a portal, with an opportunity to register and receive notifications in areas of interest. This would enable HCOs and consumers to quickly and reliably understand the process and timeline for potential patient access to a new therapy in Australia.

Recommendation 2: By principle, HCOs should be well informed to provide meaningful and compelling contributions across the lifecycle of a medicine

To enable HCOs to provide meaningful and compelling input, clear and accessible guidelines and educational resources should be developed to equip HCOs with the information and understanding needed to effectively participate in activities across the lifecycle of a medicine.

Educational programs and resources that are currently available to HCOs were emphasised as being

highly valuable, although not routinely available. Broader access for HCOs to educational programs was supported by all stakeholders.

In addition, the Department of Health's Consumer Evidence and Engagement Unit (CEEU), working with the PBAC, should provide clear guidelines for HCOs on the types of information and standard of evidence that would be of value in HTA processes and decision making. This should allow for flexibility, depending on the condition and nature of the therapy being considered.

By example, HCOs shared that limited guidance around consumer hearings including, how these are decided to be held and expectations of attendees makes it challenging for HCOs to prepare and participate in their best capacity. To increase transparency and general understanding of the reasons why consumer hearings are held for particular therapies, and not others, the PBAC should publish a documented process that details how this decision is made, and the criteria utilised to determine if a consumer hearing is needed. Noting that more recently, the PBAC have increased the use of multi-stakeholder conversations which have in some instances included consumers, the need for guidance across both engagements remains.

In advance of consumer hearings, the PBAC should share with attendees clarity and guidance on the impetus, purpose and key topics for discussion during the hearing. This is to ensure that the consumer hearing process facilitates open sharing of information, by enabling patients and their representatives to suitably prepare.

There was consensus amongst the HCOs that they would value the ability to request a consumer hearing, noting the need for clear criteria to ensure this pathway was used appropriately.

Recommendation 3: Proactively share plain language summaries across the lifecycle of medicines

Participating HCOs enthusiastically supported the Summary of Information pilot, which is currently under review by the Department of Health and Aged Care's CEEU. This pilot saw participating companies provide a Summary of Information for Patients, leveraging work of the international collaboration, Health Technology Assessment International (HTAi), to develop plain language summaries of HTA applications for HCOs and consumers in a template format that supports the provision of consistent information. The Australian pilot aimed to provide information for relevant HCOs to support their ability to contribute to HTA processes and decision making.

HCOs expressed that they would welcome the expansion of the pilot and/or more formal adoption of this initiative. However, it was noted by HCOs and other stakeholders interviewed that further work was required specific to:

- defining which therapies would benefit from such summaries;
- confidentiality and direct to consumer considerations; and
- resourcing requirements.

Importantly, it was also highlighted that this would require alignment across Industry as to their willingness to participate, followed by ongoing and active Industry engagement to support the development of the summaries at various stages across the medicines lifecycle. Medicines Australia, in partnership with the Department of Health was seen as a key stakeholder in helping to progress this initiative.

The value seen by HCOs was such that they saw transferability of the plain language concept to clinical trial protocols, TGA Australian Public Assessment Reports (AusPAR) and PBAC public summary documents.

Recommendation 4: Tailor submission forms for different stakeholders

Consumer comments can be provided to the PBAC via the Office of Health Technology Assessment (OHTA) consultation hub by responding to a series of questions.[6] There is an opportunity to enable HCOs to provide more meaningful submissions by developing a separate submission form that is fit for purpose for those providing collective, survey or cohort-based submissions. HCOs reported using "work around" processes such as directly emailing their collated comments, rather than using the standard process, while some did not know of alternative options for their submissions. A tailored submission for clinician stakeholders was also discussed and all participating HCOs were supportive of approaches that encouraged increased clinician input.

Recommendation 5: Improve feedback and the provision of information following a decision

Significant effort and resources are dedicated to the development of comprehensive consumer submissions. In recognition of this and to enable the development of well-informed consumer submissions in the future, HCOs expressed a strong desire to receive tailored feedback from the CEEU and/or PBAC on their submissions – again noting the likely requirement for additional resourcing to allow implementation.

Following each decision, Public Summary Documents (PSDs) are published for each medicine considered by the PBAC. The PSDs are publicly available on the PBS website. To complement the provision of PSDs, which are highly technical and challenging for patients and their representatives to interpret, the PBAC, in collaboration with product sponsors should pilot the provision of plain language summary documents or alternatively the provision of a summary that shares the key reasons for the decision taken. This information could be utilised by HCOs to communicate with their patient communities who can be heavily invested in the PBAC outcomes.

Broader Value Considerations

HTA deliberations typically focus on "hard" clinical outcomes derived from randomised controlled trials (RCTs). RCT evidence is recognised by regulators and HTA agencies such as the PBAC as the gold standard. RCT evidence, however, does not always capture well the outcomes and experiences that matter to patients.

HCOs and other stakeholders who participated in the focus groups support the adoption of broader perspectives in HTA decision making, acknowledging that sponsor companies and other stakeholders must work together to provide robust evidence of these broader value considerations. These broader value considerations were seen by HCOs to be of significant importance in disease areas where clinical trial data is limited, or equity issues exist.

Incorporating broader value considerations into HTA decision making was acknowledged as being complex and requiring multiple approaches from the clinical development stage through to the actual HTA decision and potentially beyond to post-marketing experiences. The role of consumers across this continuum of value assessment was recognised by HCOs.

Recommendation 6: Increased international collaborations with HCOs to influence global clinical development standards in support of evidence collection that can be utilised by decision makers in HTA

There is a growing trend for trial design to increasingly involve HCO input to ensure that the data collected is meaningful, therapy area specific and consistent.

However, decisions regarding the design and conduct of phase 3 clinical trials are typically made outside of Australia, and do not always consider the needs of international regulators and HTA bodies. Recent moves towards collaboration among HTA agencies in Australia, the UK and Canada may provide opportunities for greater alignment and action with regards to highlighting the importance of patient reported outcome measures in clinical trials.[7]

HCOs reported individual circumstances where they had been engaged by international HCOs and HTA agencies; however, greater support and guidance could enable Australian HCOs to collaborate globally and shape international trial design on a more regular and consistent basis.

Recommendation 7: A more adaptable process that allows for upfront patient and clinician input, and captures patient priorities in the evaluation of medicines

HCOs believe that it is important to influence the frame within which HTA is conducted in order for patient perspectives to really matter in the decision. This may include considering relevant quality of life measures, as well as broader implications for social and workforce participation of patients and their families or carers. To allow for collection and collation of these value constructs, this requires:

- Early notification and awareness of an upcoming HTA process in a relevant disease area.
- Plain language information to be available to consumers regarding the submission and key areas that would benefit from understanding consumer perspectives.
- Opportunities for consumer engagement early in the HTA process, where this is warranted to frame the PBAC's decision making, without unnecessarily extending overall HTA timelines.
- Guidance from PBAC (or CEEU) regarding which medicines and health technologies would benefit from additional consumer input and which value area is of interest.
- Greater formalisation of broader value impacts that can be considered in the evaluation of medicines.

Within the current HTA process, stakeholders believed that there are also opportunities for HCOs, clinicians, and sponsor companies to work together to more effectively frame HCO and consumer submissions to the PBAC. This work would need to be done before the PBAC submission and, hence, would need to not extend the review time. Barriers to sponsor and HCO collaborations were however raised as a significant issue in realising these opportunities and included concerns with off label (i.e. medicines without TGA approval) discussions, direct to consumer advertising legislation and the Medicines Australia code of conduct.

All stakeholders that were consulted believed that barriers to collaborations should be explored in the context of the current HTA review and the enhanced consumer engagement initiative and require:

- Review of current challenges and risks (both perceived and real) restricting communication between sponsors and HCOs;
- Appropriate resolution of current challenges and identification of pathways to facilitate earlier engagement between sponsors and HCOs; and
- Formalisation and endorsement of these pathways and practices to give confidence and encourage consistent earlier two-way communication between sponsors and HCOs.

Speed of Access to Medicines

Discussions around the speed of access to medicines typically led to HCOs describing the inequities facing some patient communities (especially those with rare diseases), as well as the urgent needs of those with life threatening, debilitating and degenerative diseases.

HCOs highlighted the disconnect between clinical evidence in rare diseases (small patient populations, non-comparative studies, surrogate endpoints) and the standards required by the PBAC. In order to solve for this, HCOs recommended that sponsors and the PBAC work on developing a system that would allow for early access and data generation to be used to confirm the clinical and cost-effectiveness of the therapy.

There was consensus among participating HCOs to support a pilot Early Access Program (EAP) in Australia. The recommendations below set out a potential pathway for design, implementation and involvement of patients and HCOs. There was also consensus that Australia needed to improve and formalise horizon scanning practices in order to avoid unnecessary delays and ensure that patient communities are prepared and well informed. It was noted that an enabler of this would include alignment across Industry and subsequent willingness to participate.

Recommendation 8: Review existing and previous managed access programs, and assess international early access models, with a view to developing an Australian early access program

Review Australia's current Managed Access Program (and former Managed Entry Scheme) to determine the characteristics, gaps and challenges of these programs that have resulted in the low uptake by sponsor companies. This should consider what the barriers that are preventing industry from proposing managed access programs in their PBAC submissions and for those few that have, what worked well and what needs to be considered for a future program.

In addition, assess international models for early access, particularly in Europe. This assessment should aim to uncover the strengths of these programs that should be considered in the design of an Australian model, as well as the challenges and limitations of these models that have surfaced through their development and implementation. For example, it is recognised that there have been significant challenges in managing the expansion, growth and therefore cost of NHS England's Cancer Drugs Fund. Assessing and understanding the nature of these challenges would provide important lessons for the design, parameters and implementation of an early access program (EAP) in Australia.

Recommendation 9: Develop a pilot EAP, with patient representation, independent third party management and equity of access as a core design principle

A pilot EAP should be collaborative in design, bringing together patients, HCOs, industry, the Department of Health and the PBAC to ensure that the program responds to the needs of all key stakeholders.

Determining which therapies should be prioritised for a pilot EAP presents obvious challenges. Rare diseases and/or populations where inequity of access exist were seen as potential areas, as were the criteria used for the TGA's provisional approval pathway for prescription medicines. However, participating HCOs indicated that it would be important to include consumer representation in developing criteria to assess therapies put forward.

Agreeing with the uncertainties and outcomes required from an EAP were also seen as being critical to the success of a pilot. HCOs also emphasised the importance of ensuring that patients are empowered to provide informed consent to access therapies via an EAP, including the potential

for medicines to be delisted following a provisional approval period. Patients should be considered for ongoing access or supported in transitioning off the therapy, as appropriate to their individual situation. These design principles should be clear and transparent.

A standardised framework should be developed to inform data collection methods, data analysis and processes for the re-review of therapies, following the provisional listing period.

Finally, an independent third party should be considered to manage and run the pilot program with equity of access as a core design principle of an EAP.

Recommendation 10: Establish a formal horizon scanning system in Australia

Building upon the Strategic Agreement commitment (between the medicines industry and the Commonwealth) to conduct an annual horizon scanning forum, efforts should continue with the aim of establishing a collaborative, consultative and independent system to effectively identify and impartially document relevant products and technologies, including early indicators of benefit and risk, potential to address unmet needs and value of early patient engagement and insights. This would be particularly valuable for patient populations that are underserved and where equity of access issues exist, including indigenous and remote communities.

Background

Shaping Healthcare Together and Broadening the Evidence

Shaping Healthcare Together is an annual roundtable series that is convened by Bristol Myers Squibb (BMS) Australia as part of its Broadening the Evidence project. It brings together healthcare experts, including HCOs from across Australia, representatives from the Department of Health and clinicians. BMS has convened the Roundtable for the past five years, addressing and building upon the recommendations for enhancing patient engagement in the Australian HTA process made in the Broadening the Evidence report, launched in 2019.

BMS is genuinely committed to initiatives that support the role of consumers and patients in healthcare decision making and health policy and works together with all healthcare stakeholders to drive changes that will improve the healthcare ecosystem and ultimately provide improved outcomes for Australian patients.

Development of This Paper

Shaping Healthcare Together Roundtable – November 2022

The 2022 Shaping Healthcare Together Roundtable built upon the exploration of consumer involvement in HTA decision making at previous Roundtables and focused on patient centric system reform within the context of the current HTA review and the Strategic Agreement (between the medicines industry and the Commonwealth) commitment to deliver enhanced consumer engagement in the Australian HTA process. During the Roundtable, the opportunities for reform and greater patient centricity across the entire lifecycle of access to medicines were evaluated and deliberations were framed through the question; *where do we prioritise change across the continuum of health?*

The focal areas of a medicines lifecycle included:

The clinical development phase

The regulatory and HTA phase prior to PBS access to medicines

The post reimbursement phase

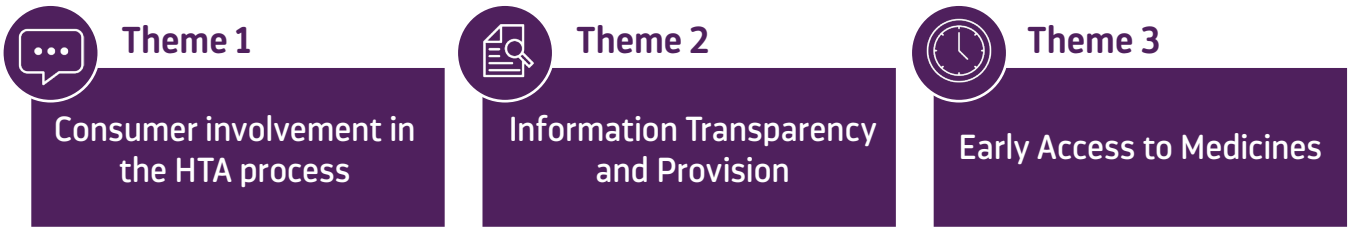
The Roundtable highlighted the genuine need for system reform, across the complete lifecycle of access to medicines and the need to underpin this with meaningful consumer engagement. In addition, acknowledging the array of activities and key events that are currently taking place in the Australian health system (2021 Parliamentary Inquiry into access to new drugs and novel medical technologies; 2023 HTA review; and 2022 Strategic Agreement Enhanced Consumer Engagement initiative), the environment presents a range of opportunities for stakeholders, across multiple levels, to collaborate, create and participate in initiatives that aim to enhance patient experience and outcomes. As such, BMS in collaboration with Biointelect continued to consult with HCOs and key government and industry stakeholders via focus groups in 2023 in order to develop this paper on potential patient centric system reform initiatives in Australian HTA.

Focus Groups with HCOs – March – April 2023

Throughout the months of March and April 2023, BMS and Biointelect held a series of focus groups with representatives from over 20 HCOs, to understand their views on what patient-centric reform of HTA may look like, and to identify practical steps towards achieving this goal.

The discussions held with HCOs across the focus groups were iterative, with each of the topics for discussion building upon one another and creating new learnings. Between the first and last focus group, the themes utilised to shape our discussions were refined based on our learnings. Across the focus groups, discussions were predominantly framed across the following three themes (Figure 2):

Figure 2: Themes explored across the focus groups



Stakeholder Interviews – April 2023

Following the focus groups, BMS and Biointelect held interviews with key government and industry representatives involved in HTA. The purpose of these interviews was to share the learnings and opportunities that were identified by HCOs and identify the practicalities associated with the opportunities from a government and industry perspective. Collating the learnings and information gathered during the focus groups with HCOs, the insights were distilled into three core themes (Figure 3) which helped frame the discussions.

Figure 3: Themes discussed across the stakeholder interviews

Building upon learnings from the Roundtable, focus groups and stakeholder interviews, this paper provides an overview of the discussions with HCOs and key healthcare experts, sharing the insights and highlighting key opportunities for patient centric system reform across the lifecycle of a medicine, with a particular focus on HTA in Australia.

Overview

An Inflection Point in Australian Healthcare Reform

The current environment presents many opportunities to engage with the Australian health system and government on reform. A revised National Medicines Policy (2022)[8] focussing on patient centricity overlays a number of key opportunities to improve patient understanding and input into healthcare decision making specific to the delivery of medicines, including:

- Recommendations from the 2021 Parliamentary Inquiry into approval processes for new drugs and novel medical technologies in Australia[9] – *still awaiting a formal government response;*
- The current review of HTA policy, process, and methods – *report due at the end of 2023*; and
- 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[10] – *work due to commence in 2023.*

Through this range of activities, a variety of stakeholders across Australia have and will provide significant input and valuable insights that can support patient centred reform. It is therefore important to continue these conversations and continue to utilise and take action on the valuable contributions made by stakeholders.

2021 Parliamentary Inquiry

The 2021 Parliamentary Inquiry into approval processes for new drugs and novel medical technologies in Australia acknowledged that there is a growing understanding among government, industry and others of the importance of the patient voice.

The Committee stated in their report that they are adamant there is a need for patients to participate in the HTA process at an earlier stage, to be equipped with more information with which to do so, and that patient feedback on their contributions to the HTA processes should be developed.[11]

The Committee's recommendations specific to the patient voice (Recommendation 28) are detailed in the text box below, with the Committee proposing that the Department make these patient voice reforms in conjunction with the review of the HTA system arising from the *Strategic Agreement 2022 - 2027* agreed between the Government and Medicines Australia.

Recommendation 28

The Committee recommends that:

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

Source: Commonwealth of Australia. (2021). The New Frontier – Delivering better health for all Australians.

The Australian Government Health Technology Assessment Review

The Federal Government is currently conducting an independent review of Australia's HTA system; the first of its kind in almost 30 years. This review is one of the main commitments under the 2022 – 2027 Strategic Agreement between the Commonwealth and Medicines Australia. Under the Strategic Agreement, there are several commitments to reforms and reform processes to ensure that Australians can:

- Access break-through medicines as early as possible; and
- Access a robust and uninterrupted supply of the medicines that are used every day.

The review is important in ensuring that the medicines assessment process in Australia keeps pace with rapidly advancing health technology and minimises barriers to access.

The HTA review will examine HTA policy and methods, whilst consulting stakeholders. There are five core objectives that centre around identifying whether key features of Australia's HTA system:[12]

1. Are working effectively;
2. May act as current or future barriers to earliest possible access;
3. May act as a current or future barriers to equitable access;
4. Detract from person-centredness; and
5. May be creating perverse incentives.

The HTA review offers an exceptional opportunity to reform HTA policy and processes with the objective of prioritising patient access to new medicines.

Enhanced Consumer Engagement Process

The PBAC is responsible for conducting HTA specific to medicines and vaccines in Australia. The PBAC is supported by a range of contracted technical review groups and subcommittees. The PBAC process aspires to be consultative and reflective of community values, as well as flexible and fit for purpose. However, the PBAC must also operate efficiently and conduct HTA consistently and objectively across the variety of medicines put forward for each of its 17-week cycles, carried out three times a year.

Significant efforts and commitments have been made recently to ensure that the consumer voice is heard and empowered to influence the decisions made by the PBAC. Recent initiatives include:

- Establishment of the Consumer Evidence and Engagement Unit (CEEU) within the Department of Health in 2019, to coordinate consumer engagement.[13]
- The Strategic Agreement between the Australian Government and Medicines Australia, commits to i) the first independent HTA Review in almost 30 years and ii) co-designing a new Enhanced Consumer Engagement Process.[14]
- Establishment of the HTA Consumer Consultative Committee (HTACCC), which acts as an advisory group, bringing consumer views into HTA processes and relevant matters.[15]

Whilst there is recognition of the recent efforts made to enhance consumer engagement in HTA processes, there is consensus amongst HCOs that patients and their representatives need to be engaged earlier and more broadly across the lifecycle of a medicine.

What is Patient Centric System Reform?

If we had a system that is more responsive to and driven by patient needs, what would that look like?

Patient centric system reform refers to making changes in the healthcare and medicines access system that allow for and ensure that patient needs are prioritised as the driving force of the system. More specifically, in the context of the current Australian HTA review and reform processes, this can be defined as;

The need for a holistic approach that prioritises patient needs, preferences and values and provides meaningful opportunities for patients to participate in and influence the reimbursement decision making process.

In the context of HTA, patient centric system reform would mean that future process improvement efforts and changes to policy would focus primarily on, or better prioritise the needs, preferences and values of patients. This approach aims to empower patients and prioritise their needs in the decision making process, to ensure that patients have faster and more equitable access to therapies that improve healthcare outcomes.

Ensuring that future reform efforts, particularly in HTA utilise a patient centred approach, enables the HTA process to become more responsive to the needs and preferences of patients, ultimately leading to better informed and more equitable decisions regarding the adoption of innovative therapies that have lifesaving and have lifechanging impacts for patients.

As described above, the current environment represents an inflection point in Australian healthcare reform and presents many opportunities for stakeholders to engage with the Australian health system and government on reform that can bring patient centricity to life. The consultations held with HCOs and government and industry stakeholders provided insights into how patient centricity could be improved in the assessment and delivery of new medicines to Australian patients. The three core themes identified through these conversations are described in detail across the following sections.

Effective And Meaningful Consumer Engagement

New therapy approvals are global news, particularly for patients seeking alternatives to their current treatment or options where there are few, if any available. Australian HCOs serve increasingly informed communities with access to globalised information, via the internet, that immediately disseminates news about approvals in the US and Europe. These patient communities can, at times, be actively seeking information about access to new therapies before they have even been submitted by sponsor companies to the TGA or PBAC in Australia.

HCOs "now often receive calls and emails from patients, alerting them to the news that an innovative therapy for their indication has been approved by the United States (US) Food and Drug Administration (FDA) overnight. Patients are increasingly questioning whether these therapies will be made available in Australia and the status of Australian clinical trials, regulatory decisions, and funding decisions." – HCO Representative

The rapid speed at which patients can access information about the development, registration and reimbursement of medicines across international jurisdictions, combined with the time required for HCOs to gather meaningful inputs, results in a greater need to reconsider when consumers are engaged across the development cycle of medicines and the type of information that is shared with them.

Earlier provision of information may have the following objectives:

- To better equip HCOs to respond to patient queries on the status of therapies;
- To reduce patient uncertainty, in the event of delays or waiting periods;
- To enable HCOs to better plan and resource, to develop submissions to regulatory and reimbursement bodies; and
- To provide HCOs with greater time to identify, contact, receive and collate input from patients and HCPs into submissions to regulatory and reimbursement bodies.

Consumer Engagement in Australian HTA Processes and Decision Making

It is widely accepted that the patient voice is integral to the HTA decision making process, due to the experience-based knowledge that consumers provide through living with a health condition and managing their healthcare. The Australian HTA Policy Framework, implemented by the PBAC and the Medical Services Advisory Committee (MSAC), includes a principle to conduct "*structured consultation with interested parties, including consumers*". The following processes currently exist to capture consumer input in the PBACs decision making (Figure 4):

Figure 4: Processes for consumer input in HTA, in Australia

Consumer submissions

- Any consumer may comment on medicines in upcoming PBAC agenda
- Allows 6-8 weeks for consumers' inputs to be lodged
- PBAC consumer representative reviews and collates for the PBAC agenda

Consumer hearings

- Consumers have opportunity for direct communication with the PBAC regarding medicines that seek PBS listing
- Participation is by invitation only, mainly between HCOs and PBAC representatives
- Only take place when deemed necessary by PBAC

Stakeholder meetings

- Convened where a submission for a medicine that treats a serious, disabling or life-threatening condition has not been recommended or deferred
- Patient group representatives and PBAC consumer representatives are invited to provide input

Source: Patient Voice Initiative. Three ways of being involved. Available at, https://www.patientvoiceinitiative.org/patient-experience-and-participation/pharmaceutical-benefits-scheme/

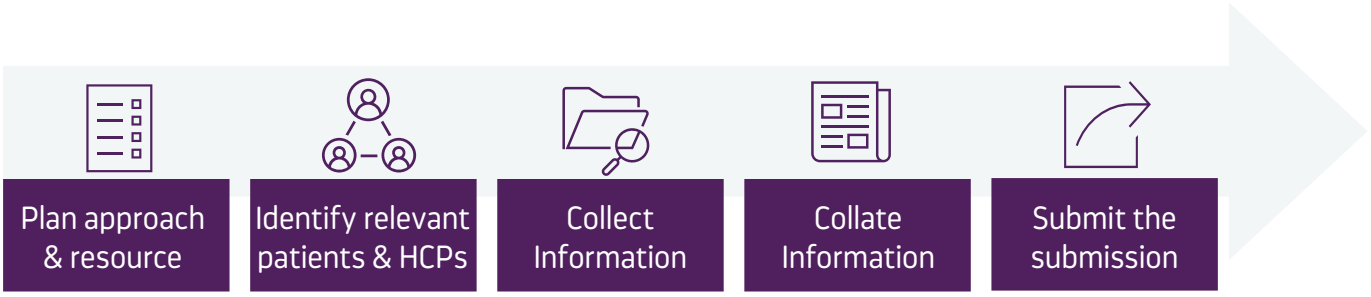
HCO Perception of Avenues for Engagement in Australian HTA Processes and Decision Making

Consumer Submissions

Process:

All consumers have the opportunity to make a comment on therapies undergoing HTA in the upcoming PBAC agenda. There was consensus amongst HCO representatives that the 6-8 week period in which they have the opportunity to lodge a submission is acceptable to produce a report. However, as HCOs quite often need to prepare, plan their resourcing, and gather input from patients and HCPs to provide meaningful input into their consumer submission (Figure 5), earlier notification was highly encouraged by HCO representatives. This is especially true for HCOs who have a large or less defined patient cohort as they may not have oversight or contact with the patient cohort relevant to the medicine being assessed.

Figure 5: Steps involved in the development of a consumer submission (HCO)



Guidance:

There is limited guidance on how to develop a meaningful submission to support the PBACs decision making. In addition, HCOs agree that it is difficult to determine the type of information that will matter to the PBAC, and that the submission form is currently tailored to input a response from an individual consumer and therefore, for HCOs that often collate input from a range of consumers to build a strong submission, the form is not suited to the needs of HCOs representing patient cohorts.

For example, a HCO representative shared that a patient had 'before and after' photos of their skin condition, following the use of a new therapy. The photo alone showcased the significant improvements in the patient's condition, which was challenging to convey through a written submission alone. The patient therefore tried to incorporate these images into their submission; however, upon submission it was evident that the PBAC does not accept the inclusion of images in consumer submissions. This issue is obviously compounded if the organisation wanted to collate and submit multiple patient images to illustrate the position.

Increased guidance along with flexibility with regards to information submitted was seen by HCOs as an avenue to improve consumer commentary.

Information to Assist Development of Consumer Submissions:

All stakeholders, including patients, clinicians and HCOs should be able to access information that is consistent and transparent, at a single location. Currently, in order to gather the information they need and the information sought by patients, HCOs have to search for and triangulate information across a range of websites and communication sources (refer to text box below). This is both time consuming and challenging to ensure that the information found is correct and up to date.

How HCOs currently access information about medicines applications and approvals

TGA

The Australian Register of Therapeutic Goods (ARTG) is an online database that is available to the public, providing information about therapeutic goods for human use approved for supply in, or exported from, Australia. All therapeutic goods must be entered on the ARTG before they can be supplied in Australia, hence making the ARTG a single source of information on all approved therapeutic goods in Australia. Information provided through the database includes the product name and formulation details and sponsor and manufacturer details.[16]

PBAC

Approximately nine weeks ahead of the PBAC meeting, whereby therapies are discussed and a decision is made, the PBAC Agenda is published and made available to the public. It is also at this time that Consumer Comments open. Approximately 16 and 18 weeks following the PBAC meeting, Public Summary Documents for positive and subsequent rejections and first time rejections and deferrals, are published to the public.[17]

Medicines Status Website

The Medicines Status Website (MSW) is available to consumers to search for and monitor the status of medicines as they progress through the PBS listing process.[18] Evident through consultation with HCOs, there is limited awareness and use of the Medicines Status Website (MSW) amongst consumers and HCOs. HCOs also noted that there is no functionality associated with the MSW that allows them to receive notifications or updates, this means that HCOs must continuously check the website for any updates.

Medicines Access Portal (MAP)

The MAP website is a joint initiative between Medicines Australia and Rare Cancers Australia, launched in 2022. It is a single, secure online portal for pharmaceutical companies to inform Australian medical practitioners of the existence of special oncology access treatment programs available to oncology patients.[19] The MAP is currently available only for cancer treatments and access is limited to HCPs.

Relationships between HCOs and industry, PBAC and CEEU representatives currently act as the best information channel for some HCOs to hear about new therapies and receive feedback on their submissions. It is, however, acknowledged that formal processes and transparency should be improved to cease reliance on relationships with sponsor companies and PBAC & CEEU representatives.

Two-way Communication:

Transparent and effective communication should include two-way communication between medicine sponsors and HCOs. Currently, effective two-way communication is often prevented by restrictions and perceptions of the restrictions around contact between sponsors and consumers, whereby sponsors are often deterred from communicating directly with HCOs due to company interpretation of the Code of Conduct. It is however evident that under Part C, Section 11 of the Medicines Australia Code of Conduct that, *"Companies are permitted to communicate proactively or reactively with relevant stakeholders, provided that discourse is limited to information that may assist the stakeholder in their role."*[20] Furthermore, the communication should be non-promotional in nature and provided by someone appropriately qualified. Noting that acting within these guidelines, communication between sponsors and HCOs is permitted, efforts should be made to clarify these restrictions and encourage communication that supports and equips HCOs to make meaningful contributions to HTA (amongst other things that serve HCO patient communities).

Feedback:

There is currently no tailored feedback provided by the PBAC on consumer submissions. There is strong consensus amongst HCOs that the absence of feedback makes it challenging to improve future submissions. Many HCOs explained that it has only been through the experience of developing numerous submissions that they have drawn patterns that have allowed them to progressively improve their submissions. Furthermore, HCOs shared that relationships and connections with industry and department representatives are important to gaining some form of feedback on a submission. It was, however, widely agreed that feedback should be formalised and reliance on relationships and connections can perpetuate inequity. The lack of guidance pre submission and feedback post submission were highlighted as key challenges for HCOs understanding what information would be most useful to the PBAC.

Following each decision, the PBS releases PSDs which are standardised documents that aim to inform the public of PBAC decisions and the basis of each outcome.[21] There was agreement amongst HCOs that patients and their representatives experience difficulty in understanding and interpreting PSDs, due to their technical nature. In addition to providing HCOs with feedback on their submission, plain language summaries of PBAC decisions should be developed and disseminated for public access. This will allow patients and HCOs the opportunity to comprehensively understand the decision and basis for each PBAC outcome.

Consumer Hearings and Stakeholder Meetings

Consumer hearings are held by the PBAC, when deemed necessary for the therapy being assessed. Attendance at a consumer hearing is by invitation only, however, for the HCOs or consumers invited, they have the opportunity for direct communication with the PBAC regarding the therapy that is seeking reimbursement.

The majority of HCOs are aware of the potential to engage with the PBAC through consumer hearings, with numerous HCO representatives reporting that they have requested that the PBAC hold a consumer hearing for a therapy being assessed. It is however apparent that there is currently a lack of transparency regarding how the PBAC makes the decision on whether to hold a consumer hearing or not. This presents the opportunity for the PBAC to publish a documented process for how the decision is made and the criteria utilised to determine whether or not to hold a consumer hearing.

Several HCO representatives reported that they have been directly involved in a consumer hearing, through attendance and participation. HCO representatives all similarly expressed that participation in a consumer hearing had been a daunting experience for themselves and especially, for the patients attending. This can be attributed to a number of reasons including:

- Limited guidance and information, such as a detailed agenda, is provided ahead of the hearing, meaning HCOs and patients have little understanding of the PBACs expectations or what they themselves should expect.
- HCOs and patients are not provided with an indication of the types of questions to prepare for ahead of the hearing.
- HCOs acknowledged that it is an intimidating experience for a patient to share their personal experience with a health condition, to a panel of PBAC representatives in a formal setting.

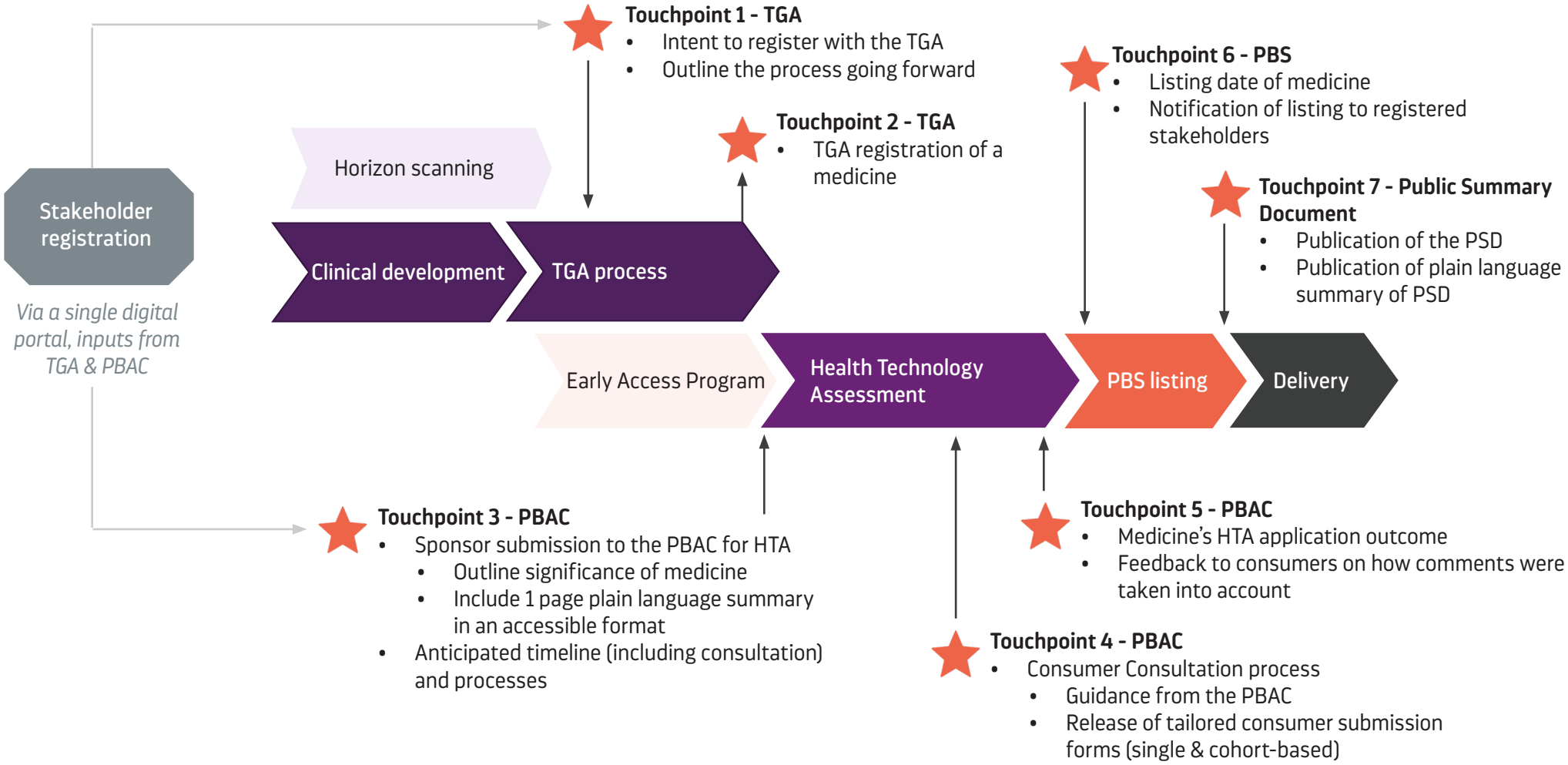
HCOs and patients have a tremendous sense of responsibility to adequately represent other patients and can feel accountable when a decision by the PBAC to not recommend a medicine is taken.

The consultative process led to the development of **ten** recommendations to support patient-centric system reform. The recommendations for effective and meaningful consumer engagement in HTA processes and decision making are on **pages 7 – 9**. In addition, Figure 6 provides an overview of the touchpoints that participating HCOs agreed were important for effective communication and engagement with patients and HCOs.

Improving consumer engagement across the medicines and health technology development continuum

The model, below, provides an overview of the touchpoints that participating HCOs agreed were important for effective communication and engagement across the medicines lifecycle.

Figure 6: A model for proactive communication and engagement with HCOs



Broader Value Considerations

Australia's national HTA processes cover all medical technologies where a listing on the PBS or National Immunisation Program (NIP) is sought. Listing on the PBS ensures subsidised, affordable, and equitable access to medicines for Australian patients. It is especially important for innovative medicines that have the potential to deliver significant improvements in quality of life and health outcomes.

PBAC Guidelines focus primarily on the direct impacts of new medicines; i.e. clinical outcomes for patients and resourcing implications for the health system and the health budget. While the PBAC accept and review evidence regarding indirect, or broader effects, these factors are not always influential in decision making. This may include, for example, impacts of caring for a patient on families or carers.

The HTA process is initiated by a sponsor submitting an application to the PBAC. Although it is possible for a HCO to make an application, this is unusual in practice. The very nature of this process means that the system is typically reactive, rather than proactive to patient needs, with little ability for patients and their representatives to influence which medicines patients can access through the PBS.

Patient Input Into How Value Should Be Defined in HTA

HCOs agree that it is important to consider factors beyond clinical outcomes in HTA decision making. In line with the concept of patient centric system reform, incorporating a patient centred approach in HTA, allows for a broader interpretation of the impact of therapies on patients' health, well-being, and lifestyle. This would also allow for more flexibility, to consider and incorporate indirect impacts and diverse factors that matter most to patients in the decision making process.

Parameters of Decision Making

The HTA process must be objective and accommodate a range of diverse conditions, therapy types and modes of administration. In order to make an objective assessment, HTA must utilise standardised methods to prioritise medicines for PBS listing. Medicines are evaluated on the basis of their clinical benefit and cost-effectiveness, as demonstrated in RCTs, when compared to the standard of care for the same condition. Outcomes which are of great value to patients, such as improvements in quality of life, are often not well measured in RCTs, and therefore may not be weighted accordingly by decision makers and accurately accounted for in the HTA process.

It is important to think holistically and across different cohorts about patient access and experience in relation to medicines. The HTA process results in challenges in representation, whereby the nature of the standardised process means that the detail of individual experiences may become less influential. Outcomes that are important to individual patients or patient cohorts, such as improvements to quality of life due to a new formulation or route of administration, may be devalued when they are incorporated into an objective and quantifiable measure.

Patient centred reform requires flexibility, allowing patients to submit information with significant importance to them at a point in the process that enables this to inform the parameters of decision making. Currently, when patients provide information that does not fit within the established parameters of decision making, the information may be distilled into a standardised process that may devalue its significance or mean it cannot be considered.

HCOs and individual consumers can be a critical source of relevant information to inform these parameters, such as:

- Quality of life impacts, from the patients' perspective and beyond those currently considered;
- Productivity, including the ability for the patient and/or family members to work;
- Social impacts, including the ability to attend familial milestones, socialise, travel; and
- Functional improvements and/or delay in health condition progression.

By incorporating these broader considerations within the HTA value construct, it would also place a greater onus on sponsor companies to gather and report robust data for these factors.

Considering a deliberative approach to HTA decision making to allow patient input to inform the parameters of decision making

Deliberation in HTA is the informed and critical examination of an issue and the weighting of arguments and evidence to guide a subsequent decision, as defined by a joint Health Technology Assessment International (HTAi) and International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Task Force. A deliberative process is structured to enable patients and their representatives to provide actionable input on what is discussed in decision making.

HCO feedback on important evidence and factors that should influence PBAC decisions could inform a criterion that can be utilised in a deliberative process in Australian decision making. Following a decision, outcomes of the deliberative process could be fed back to patients in a criteria or assessment matrix, in plain language summary documents.

Source: ISPOR. (2022). Global Expert Panel Publishes Guidance on Deliberative Processes for Health Technology Assessment.

Gathering Better Data: Patient Valued Outcomes

From a patient perspective, the experience of living with a health condition is broader than the medical implications associated with it. Therefore, when assessing the safety and clinical effectiveness of a therapy, without discounting these factors, greater emphasis should be placed on patient reported outcomes and other factors that are deeply important to the well-being of patients, such as quality of life, the ability for themselves and family members to work, the ability to attend family milestones and socialise.

There are opportunities to better utilise quality of life data in the HTA decision making process. Despite efforts to align reporting standards, it is evident that the collection of quality of life data in clinical trials remains a challenge due to difficulty in operational implementation, inconsistent adherence to reporting standards and a lack of consensus on how to analyse and utilise the data collected.[22]

Phase 3 clinical trials are typically global and their design needs to better consider the requirements of international regulators and payers. Globally, trial design increasingly involves HCOs, to ensure that meaningful, therapy area specific, and consistent data is collected. More could be done in this regard.

Consulting patients and HCOs to supplement RCT data with their own perspectives is challenging. Unless patients have been part of a clinical trial, they are unlikely to have lived experience of a therapy being considered by the PBAC. Hence, they may provide valuable information about the impact of the condition on their daily lives (disease burden) but may not be well placed to comment with certainty about the potential impact of the therapy on alleviating that burden.

Integrating Equity Considerations into Decision Making

Equity is a decision factor for the PBAC, implying that medicines that have significant impacts for underserved populations should be prioritised. This may include those that face barriers accessing healthcare services, lack knowledge of the health system and/or face shortage of available providers and healthcare services.

Potential access issues are often not visible in the HTA process. A patient in a rural or remote community may be impacted by a change in the route of administration of a therapy, from a clinically administered intravenous (injected into a vein) to a self-administered subcutaneous (injected under the skin) therapy. This may avoid the patient traveling to a clinic to access the therapy, resulting in better access and alleviating the financial and social burden that is often placed on patients who live in rural and remote locations.

Considering disease burden across these different groups of patients is important to seeing these potential impacts of new treatment options. These factors must then also carry weight in the HTA decision making process.

Incorporating broader value considerations into the lifecycle of a medicine and in particular, HTA is a complex feat that requires multiple approaches from the clinical development stage to HTA decision making, underpinned by input from stakeholders at all levels.

The consultative process led to the development of **ten** recommendations to support patient-centric system reform. The recommendations for broader value considerations in HTA processes and decision making are on **pages 10 – 11**.

Speed of Access to Medicines

In Australia, funded access to prescription medicines by the Commonwealth Government, through the PBS is required for most patients to access innovative therapies reliably and affordably. Following a successful sponsor application to the TGA to seek approval to market (sell) a new therapy in Australia, an independent expert group, the PBAC, evaluates the therapy to determine its cost effectiveness and provides a recommendation to the Government as to whether reimbursed access should be provided through listing on the PBS.

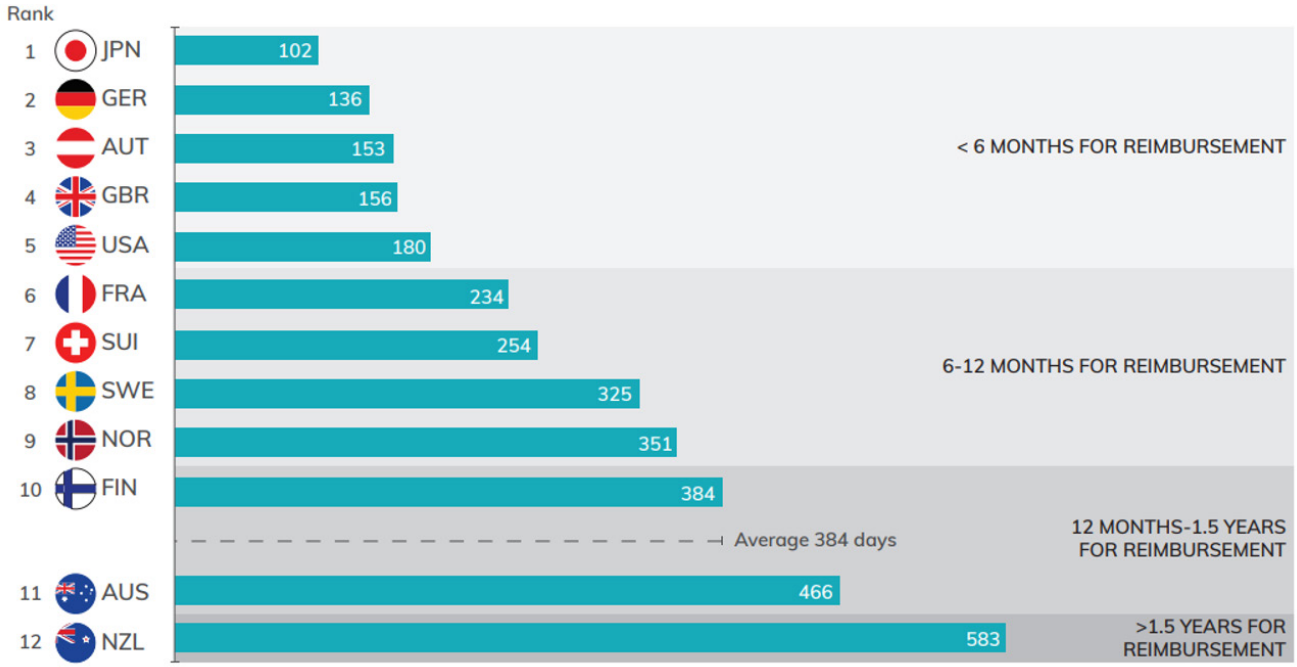
The PBAC process is held after TGA approval of the therapy, or in some cases parallel to TGA assessment, through the TGA-PBAC parallel process that allows sponsors (generally a pharmaceutical company) to submit Category 1 and Category 2 submissions to be evaluated and considered by the PBAC at any time following the lodgement of a TGA registration dossier.[23]

The HTA process occurs across a 17-week period, however, the time to access a medicine is significantly longer and there is often the need for multiple submissions to the PBAC particularly for novel medicines with a unique mechanism of action that has not previously been considered by the PBAC.

While the most recent data from the Department illustrates that the average time taken from receipt of PBAC minutes (i.e. following the completion of the HTA process and a positive recommendation) to PBS listing for medicines listed in 2021 - 2022 was 101.8 days (3.4 months),[24] the most recent analyses specific to the time taken from registration to reimbursement of First-in-Class therapies demonstrates that Australia, at 466 days, is below the OECD average.[25]

It is important to note that during the focus groups, it was highlighted that First-in-Class therapies are those that patients are often wanting as they deliver the greatest benefit in areas of high unmet clinical need.

Figure 7: The average time from registration to reimbursement for NMEs in OECD nations from 2016 - 2021



Source: Medicines Australia. (2022). Medicines Matter 2022. Australia's Access to Medicines 2016 - 2021, Figure 5

Recognising recent improvements made by the DoHA and Medicines Australia with regards to reducing time from receipt of PBAC minutes to PBS listing, it is evident that additional efficiency gains across the continuum of access to medicines are needed. From registration to subsidised listing, issues such as delays between TGA and PBAC processes, resubmission churn and post PBAC processes mean Australian patients are missing out on or waiting significant periods to access therapies that are potentially lifesaving or life-changing.

Early access to potentially transformative medicines is a universal challenge. Early access models have been implemented in other countries, notably France and England, in order to prioritise patient access, through interim funding arrangements, before rigorous HTA is conducted. These models complement provisional approval pathways through regulatory agencies and may be a solution to provide equitable early access to innovative medicines in Australia.

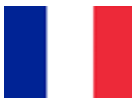
A review of international models of the existing mechanism in Australia, together with ideas from HCOs and key stakeholders as to how to improve and involve patients in early access programs are expanded upon below.

Learnings from International Models and Australia's Managed Access Program

Globally, there are numerous examples of models to facilitate early or interim access to medicines, underpinned by the objective of enabling earlier patient access to new and innovative medicines. Eligibility criteria focus on targeting unmet needs and areas of major public health interest. International models of note include:

- France's Autorisation d'accès précoce (refer to Table 2);
- NHS England's Cancer Drugs Fund and the Innovative Medicines Fund; and
- Germany's AMNOG process which provides automatic reimbursed access for all patients whilst HTA is conducted.

Table 2: Case study example of early access (accès précoce) and compassionate access (accès compassionel) in France

France's Autorisation d'accès précoce			
	France's Early Access Authorisation (APP) scheme allows reimbursed access to therapies indicated for severe, rare, or incapacitating conditions. APP applies to therapies that have not yet been granted regulatory approval, as well as those with regulatory approval, that haven't received reimbursement yet.		
	AP (accès précoce; early access) *Drugs that aren't registered in France*	**AC (accès compassionel; compassionate access)** *Drugs that are registered but not for the patient's condition*	
		Compassionate use	**Compassionate prescription**
Description	• Early access to drugs for serious, rare and disabling diseases, at the request of the manufacturer	• Access to drugs not intended to be marketed, at the request of the prescriber	• Access to drugs not intended to be marketed *in that indication*, at the request of the regulator (ANSM - Agence nationale de sécurité du médicament et des produits de santé)
Coverage	• Pre-marketing authorisation • Post- marketing authorisation (for the gap between pre-marketing authorisation and full listing)	• Compassionate use authorisation	• Compassionate prescription scheme
Eligibility criteria	• There is no appropriate treatment • The initiation of the treatment cannot be deferred • The efficacy and safety of the medicinal product are strongly presumed based on the results of clinical trials • This medicinal product is presumed to be innovative, notably compared with a clinically relevant comparator	• Product not marketed in France • There is no appropriate treatment • Efficacy and safety presumed	• Product not marketed in that indication in France • There is no appropriate treatment • Efficacy and safety presumed
Duration	• 1 year, renewable	• 1 year, renewable	• 3 years, renewable
Pricing	• Free pricing set by manufacturer	• Annual flat price per patient or set by manufacturer	• Same price as product for in-market indication
Rebates	• Provisional rebates defined by a progressive scale of turnover • Mandatory rebates may apply if there is a delay in the reimbursement submission	• Price cap may apply (rebates for amounts above cap)	
Data collection	• Manufacturer must agree to fund data collection meeting the specification of the HAS and ANSM		
Decision time	• 80-day target		

Source: Biointelect adaptation from Haute Autorite De Sante (HAS), Authorisation for early access to medicinal products: HAS assessment doctrine 2021.[26]

There is recognition amongst HCOs and key government and industry stakeholders that whilst these international programs should be understood and could be utilised as a guide to the implementation of a program in Australia, an Australian model for early access should be locally relevant and tailored to meet the needs of Australian patients and the characteristics of the Australian health and HTA system.

HCOs and other stakeholders support conducting an assessment to determine the advantages and disadvantages of Australia's Managed Access Program and international models for early access, to ensure the learnings from these make for a more equitable, efficient and sustainable program in Australia.

In Australia, the Managed Access Program (MAP), formerly the Managed Entry Scheme (MES), is a mechanism that enables a submission that would not normally be recommended for listing by the PBAC due to unacceptable clinical and/or economic uncertainty, to be listed, provided the MAP parameters are met.

The PBAC provides advice to the sponsor on initial sources of uncertainty and whether the evidence provided in the submission is sufficient to support listing under this program until a final review of additional evidence is completed, at a predefined point in time. Through the MAP, patients can access medicines earlier whilst the sponsor seeks to enhance the quality and strength of evidence provided to decision-makers in subsequent reimbursement applications.[27]

Under special circumstances, the PBAC may agree to subsidise the cost of a medicines on a provisional basis. This is enabled through the MAP which allows the PBAC to grant interim access for medicines where:

- Patients have a high and urgent clinical need.
- There is enough evidence to show the benefit of a medicine, but not enough to demonstrate cost-effectiveness.
- The sponsor will be able to provide more evidence in a reasonable time period so the PBAC can undertake a full assessment of its value.

Despite this mechanism, the MAP does not guarantee that a medicine will be listed on the PBS, as the PBAC may recommend "de-listing" based on further assessment of the efficacy and safety of the medicine. Furthermore, historically, the MAP has been utilised as a pathway to reimbursement for medicines that have undergone multiple resubmissions, due to challenges in reaching a price agreement. There has also been limited transparency on the use and parameters of the MAP and reports that there have been challenges in monitoring or managing agreements such as data collection agreements that have been utilised to enable managed access. Due to these challenges, the Managed Access Program is not a consistent or frequently utilised means to provide Australian patients with early access.

Support for an Early Access Program in Australia

There is recognition amongst HCOs and key government and industry stakeholders that whilst these international programs should be understood and could be utilised as a guide to the implementation of a program in Australia, an Australian model for early access should be locally relevant and tailored to meet the needs of Australian patients and the characteristics of the Australian health and HTA system.

HCOs and other stakeholders support conducting an assessment to determine the advantages and disadvantages of Australia's Managed Access Program and international models for early access, to ensure the learnings from these make for a more equitable, efficient and sustainable program in Australia.

There is widespread support amongst HCOs and key government and industry stakeholders for a program that enables patients to access innovative therapies more rapidly in Australia. HCOs are supportive of a transparent program or pathway for early access and agree that it is critical to enable faster access to

medicines that are potentially lifesaving or life-changing, with the potential to prevent or delay the onset of irreversible disease progression or disability caused by degenerative conditions.

In principle, HCOs should be involved in:

- Design of early access arrangements, including in the design principles at the heart of a future program and for data collection protocols. This will ensure that the program is designed with patient needs at its core and that there is equity amongst all patients, with representation of those who are often underserved.
- Prioritisation of medicines and conditions for early access. For example, it is important that HCOs are involved in setting out the criteria by which products are assessed against to be made available for early access.

There is an opportunity to utilise international evidence to facilitate earlier access to medicines. Early clinical trial data and real world evidence (RWE) from comparable international jurisdictions may provide a degree of confidence around the safety, efficacy and potential of a therapy, which can support the decision to provide early access. It is also important to recognise the diversity and unique characteristics of the Australian population.

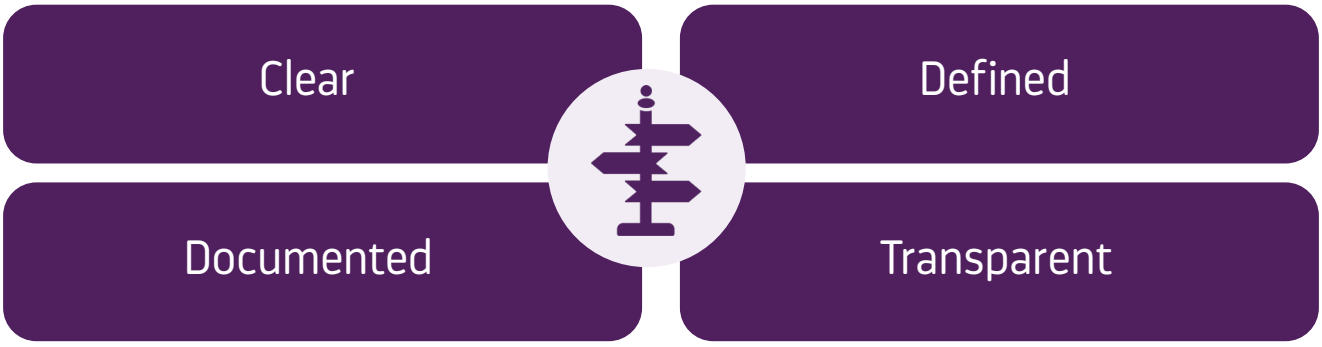
Most HCOs consulted indicated that an early access model could create value by gathering local RWE about the safety and efficacy of a therapy. In particular, for indications that a therapy is not currently listed for, an EAP could enable the generation of evidence that may support the expansion of the listing to other indications.

Important considerations

Contingent to the support amongst HCOs for an early access program is the need to establish clear parameters around the program and principles around transparency, eligibility criteria, patient involvement and decision making. Important considerations span the development of a program and throughout the lifecycle of patients accessing medicines via an EAP.

Process and Decision Making

In order to ensure that an EAP facilities equitable access to all individuals despite their condition, socioeconomic status, geographical location or race, a transparent process for funding products through this mechanism must be established, with contribution from HCOs. For all stakeholders involved, the decision-making process and criteria for funding products through an EAP must be:



Criteria for considering the funding of medicines through a newly introduced EAP should be clearly documented, with commitments to review on a regular basis. There should also be transparent and clear protocols documented for the event that a therapy is delisted, following HTA. Clear guidelines should be developed to communicate the options available to HCOs and patients following delisting and the process for transitioning a patient off the therapy, if required.

Transparency

Patient access to innovative therapies that are funded through an EAP should be equitable. Currently, the majority of Australians have mostly equal access to healthcare and medicines through Government schemes such as Medicare, the PBS, the Repatriation PBS and the Life Saving Drugs Program (LSDP). Despite initiatives and policies that intend to enable equitable access, some population groups have lower levels of access in practice due to living in rural and remote areas, their cultural background and socioeconomic status. Given these issues persist for existing programs, increased efforts are required to address these in the context of an EAP.

During the introduction of the EAP and throughout its life, information on how patients can access medicines through this program should be made available and accessible to all Australian patients, HCOs and healthcare professionals (HCPs). Clinicians and HCOs should work together to enable this information to be disseminated to patients and to support patients, should they need to travel or have any barriers to accessing treatment.

Patient Involvement

Patients that access a medicine through an EAP must be well informed about the therapy, any uncertainties in the safety and efficacy of the therapy, as well as the EAP process and alternate options for their care. If patients will be involved in a post-marketing study, then the requirements and responsibilities of this must be clear. Patient involvement in an EAP must be underpinned by informed consent, whereby patients understand the evidence supporting use of the therapy and characteristics of the EAP model.

Figure 8: Principles underpinning patient involvement in an EAP

Where RWE studies are conducted, data collection requirements may be similarly stringent to those of a clinical trial, and may, for example, prevent a patient from being able to travel whilst utilising the therapy. Such requirements should be clearly communicated to patients prior to enrolment. In addition, patients involved must understand how their data and information on the outcomes of their care will be stored and utilised in the future.

A Pilot Program For Early Access in Australia

There is consensus among HCOs and key government and industry representatives that a pilot program should be designed to trial the introduction of an early access program in Australia. A pilot program for early access must be proactive in addressing the needs of Australian patients and tailored to the system that enables access to medicines in Australia, including regulatory and reimbursement processes. This pilot should be designed through collaboration between patients, their representatives, decision makers and industry. It was suggested that this pilot should be overseen and run by an independent stakeholder to ensure that the program is managed independently and objectively. This independent party could be an entity such as an academic group, with the capabilities to collect, store and interpret data sets to ensure that RWE is collected and interpreted effectively, efficiently and accurately. The recommendations on pages 12 - 13 set out a pathway for designing and implementing a pilot early access program in Australia.

Horizon Scanning

There was agreement across HCOs in the focus groups and other key stakeholders across government and industry that horizon scanning is an important component of allowing for planned and early access to medicines in Australia.

Ultimately the goal of horizon scanning is to ensure that there are no unnecessary delays to patients accessing the medicines and health technologies they need.

While it is recognised that horizon scanning activities are already being undertaken informally by stakeholders across the healthcare ecosystem in Australia, including by HCOs, these activities are highly fragmented and there is significant variation in the approaches taken. This is inefficient and leads to significant duplication of effort, particularly where public information regarding the design, progress and results of global clinical trials is the main data source.

Formalised horizon scanning processes have historically been designed primarily to inform payers, regulators and healthcare providers; though, some institutions recognise that a broader range of stakeholders have the potential to effectively contribute to, and benefit from, this information and analysis.

For patients and caregivers, awareness and understanding of potential future health technology launches would enable better preparation and therefore more thorough and impactful contribution to HTA and reimbursement processes together with better preparedness for services and resources required. Information gathered through horizon scanning may also be utilised to raise awareness about clinical trials in Australia.

Building upon the Strategic Agreement commitment (Clause 6.2.2)[28] to conduct an annual horizon scanning forum, efforts should continue to establish a collaborative, consultative and independent horizon scanning system in Australia to effectively identify and impartially document relevant products and technologies, including early indicators of benefit and risk, and a medicines potential to address unmet clinical and patient needs.

The consultative process led to the development of **ten** recommendations to support patient-centric system reform. The recommendations for speed of access to medicines are on **pages 12 – 13.**

Conclusion

Patients and consumers have never been better informed and empowered to understand and manage their healthcare needs. While important advances have already been achieved in involving consumers in HTA decision making in Australia, stakeholders acknowledge (and HCOs expect) that there is a need to strengthen and broaden the patient voice in healthcare decision making.

The time is right to harness the voices of patients in order to evolve a health system that can achieve better access, equity, responsiveness, and efficiency for all Australians. Australia's HTA process is currently under review; a Parliamentary Inquiry into the approval processes for medicines in 2021 recommended earlier and broader patient engagement within the HTA decision making process; and an enhanced consumer engagement process will be delivered, fulfilling the commitment within the 2022 Strategic Agreement between the Commonwealth and the medicines industry.

HCOs were involved in a Roundtable and focus groups convened by BMS and Biointelect to discuss priorities and reform opportunities that would bring to life the concept of patient centricity in healthcare decision making. In addition, consultations were held with key government and industry representatives to socialise the priorities and opportunities identified and to build upon the practicalities from a government and industry perspective.

This consultative process drew out three central themes and ten recommendations, to enhance consumer engagement, incorporate broader value considerations in the evaluation of new medicines and improve the speed of access to innovative medicines.

The recommendations include both short and medium term steps that would build upon the current consumer engagement processes in Australia's HTA decision making process and improve consumer involvement across the lifecycle of a medicine.

HCOs also recommended that they be involved in more ambitious, broader reforms across the lifecycle of a medicine, such as the establishment of a formal horizon scanning system, inclusion of a broader value construct within HTA decision making, and the piloting of an early access program for innovative medicines to ensure speed of access for Australian patients.

HCOs and other stakeholders felt that many of the recommendations could be considered within the suite of initiatives and reviews currently being undertaken. However, it was noted that for true reform and patient centricity to occur, a partnership approach across Government, Industry and HCOs is required and that while structural changes to processes can support patient centricity, a wider lens would be needed to ensure consideration of associated policy and legislative changes that may be needed.

Unequivocally, effective and sustainable reforms to Australia's healthcare systems, must begin with the patient at the centre.

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