Biointelect

BROADENING THE EVIDENCE

Enhancing patient engagement in health technology assessment of medicines in Australia (with special consideration of oncology medicines).

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Executive Summary

Australia's national health technology assessment (HTA) processes include the assessment of all prescription medicines where a listing on the Pharmaceutical Benefits Schedule (PBS) is sought. Listing on the PBS ensures subsidised, affordable and equitable access to medicines for Australian patients. It is especially important for high cost new medicines that may deliver significant improvements in health outcomes for areas such as cancer and rare or complex conditions.

This HTA process is conducted by the Pharmaceutical Benefits Advisory Committee (PBAC), supported by a range of contracted technical review groups and subcommittees. It is established in the context of the National Medicines Policy, which includes the objective of equitable and affordable access to medicines. HTA has been part of the PBAC process for 25 years, with this early introduction and long-standing application resulting in Australia being regarded as an international leader in the field.

The PBAC process aspires to be consultative and reflective of Australian community values, as well as flexible and fit for purpose. However, at the same time, it is necessary for the PBAC process to operate efficiently given the number of manufacturer submissions received for each of its three 17 week cycles every year.

Note that patients and patient advocates are termed 'consumers' in the Australian HTA processes. We have used this term when referring to the Australian processes. However, because most other jurisdictions and most literature in this area uses the term 'patients', we have also used this term when referring to HTA processes outside Australia. We regard the terms as interchangeable.

Over the past 10-15 years there has been an evolution in the way in which consumers can engage and participate. While the evolution has been welcomed, there is a general sense among stakeholders that the processes for engagement and participation could be further improved, to really do justice to the consumer perspective and to broaden the evidence considered by the PBAC. Internationally (including in Australia), there has been a move to accelerate regulatory processes and timelines. While this is seen to be beneficial in terms of access for patients, it brings an increase in uncertainty regarding the quantity and quality of evidence required for HTA purposes. Consequently, the patient / consumer perspectives are even more critical, as they can contribute to addressing that uncertainty.

BMS Australia commissioned Biointelect to research the perspectives of Australian stakeholders to obtain insights on the experiences of consumers and views on areas for improvement. This was done via desk research, in depth stakeholder interviews, an on-line survey and a workshop involving several advocacy organisations.

Biointelect also interviewed a range of experts associated with patient / consumer engagement in other HTA jurisdictions, notably England, Scotland and Canada. While it is not the intention of this report to argue that Australia's HTA process should look like any of these, it was clear that some of the issues and frustrations experienced in Australia could be improved upon by learning from some of the international practices and examples.

While most of the challenges and areas for improvement are general in nature (i.e. independent of the type of medicine being reviewed), there are some issues that are more apparent for oncology medicines. The report considers this also.

Overall, the report concludes there are several areas where patient / consumer engagement and participation could be improved in Australia. These are not necessarily new ideas – some have been identified generally via other efforts. However, this report has drawn on the experiences and examples from other jurisdictions and provides more detailed recommendations.

Additional resources, at both the Department of Health (DoH) and PBAC level, will be required in order to progress, and ultimately achieve, the PBAC goal of being consultative and reflecting the values of the Australian community.

Recommendations:

This report makes 9 recommendations for consideration (**Table 1**). As with any complex process involving numerous diverse stakeholders, some of these recommendations are easier and more straightforward to implement than others. Some would require longer time frames and extensive consultation with the range of stakeholders; whilst others may be suited to a pilot approach (**Fig. 1).** For this reason, the report uses a matrix approach, where the recommendations are grouped according to extent of the potential reward or impact versus degree of difficulty in implementation.

Table 1. Summary of 9 Key Patient Engagement Recommendations and Associated Difficulty of Implementation

Recommendations	Difficulty Implementing
1. The use of e-alerts to advise interested stakeholders of a product entering the PBAC process	Easier / short-term
2. Prompts for submission deadlines	
3. Feedback on patient submissions	
4. Consumer-friendly public summary documents	Medium / longer term
5. Master classes in HTA and PBAC processes	
6. Valuation of evolving cancer survival outcomes	
7. Information on the products, provided to advocates by the manufacturer or via an independent third party	Hardest / pilot approach
8. Inclusion of advocates in a technical consultation prior to the PBAC meeting	
9. Horizon scanning	

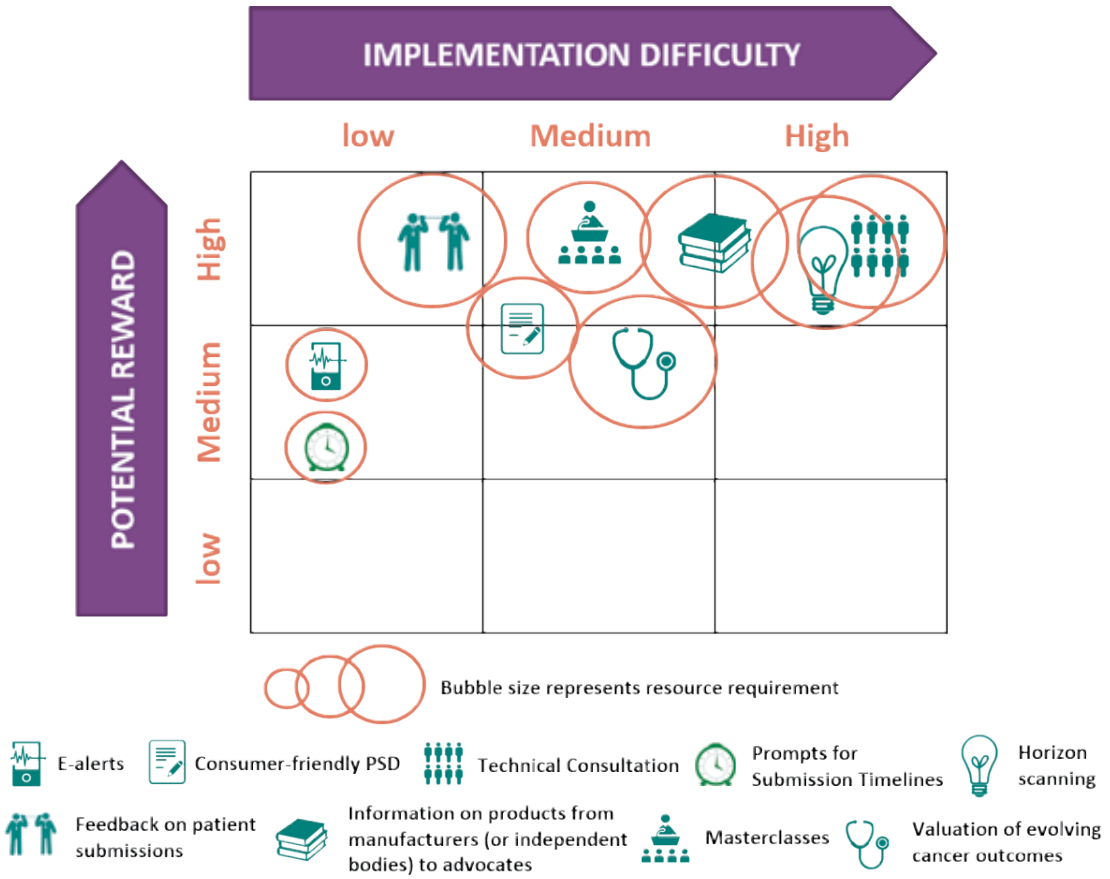


Figure 1: Matrix Representation of Recommendations for Patient Engagement in HTA

Project Background

In Australia, obtaining reimbursement of new medicines under the Pharmaceutical Benefits Scheme (PBS) requires consideration by the Pharmaceutical Benefits Advisory Committee (PBAC). The process requires the manufacturer and holder of the marketing authorisation, to develop and lodge submissions to the PBAC according to published guidelines and processes. This process is generally referred to as health technology assessment (HTA).

Australia is a world leader in the application of HTA to inform reimbursement decisions for pharmaceuticals and medical devices. Pharmaceuticals are assessed by the PBAC and associated technical review groups and subcommittees. On completion of the assessment the PBAC makes recommendations to the Minister for Health with regards to inclusion, rejection or deferral of new medicines on to the national PBS. The National Health Act specifies that such recommendations must consider the comparative effectiveness, safety and cost-effectiveness of new medicines in relation to other treatments already included on the PBS.

Over time, the 17-week cycle of assessment and appraisal has evolved to include numerous elements and interactions. One area is the way in which patients, care givers, patient advocacy organisations and others effected by a disease can have input into the process. In 2019, there are many positive elements to the process of patient engagement.

Medicines for the treatment of cancer and a range of other diseases have become increasingly complex, requiring equally intricate assessment. This has been driven in part by significant advances in our understanding of the cellular biology and genetics of cancer and other serious diseases, which has in turn lead to a dramatic proliferation of innovative treatments. These innovative treatments, while delivering significant health outcome advances, come at a high cost. As such, rigorous assessment of value is required. However, this assessment has been complicated recently by the evolution of traditional clinical trial design and the global move towards accelerated and/or provisional regulatory processes - adding greater uncertainty than usual to the HTA decision making process.

Bristol-Myers Squibb

Bristol-Myers Squibb (BMS) have a portfolio of medicines (both on the market and in development) for the treatment of a range of cancers and other serious diseases. BMS is committed to a patient-centric perspective with regards to its medicines and the diseases those medicines address. Part of this commitment is ensuring that the voice of patients and care givers is considered in HTA processes whenever they occur and especially in Australia's PBAC review process. While much progress has been made in Australia regarding processes by which patient and care giver perspectives can be included in the PBAC's deliberations, there continues to be a lack of understanding (by some advocates and patients) of the details of these processes. In addition, many patients, care givers and patient advocates are unclear as to what type of input is considered useful by the PBAC, in what form it should be presented, and whether previous input has been of value.

Internationally there is a trend to identify and support two separate but related elements: firstly, the processes and mechanisms by which patients, care givers and advocates can participate in the

HTA process (referred to as participation), and secondly, mechanisms by which patient perspectives and experiences can be researched and collated for the purpose of including them in the HTA process as more quantitative evidence (referred to as research). Most of the insights gained through this project relate to participation. The topic of how research might also be improved was also raised but may benefit from further exploration of this area in the future.

Based on a combination of these factors BMS Australia commissioned this report, to:

- engage with Australian and international HTA practitioners and stakeholders,
- develop insights into what is working well and what obstacles exist, and
- make recommendations as to what could be improved in Australia's HTA processes for medicines regarding patient and advocate participation.

This report is being made available to all individuals and organisations who contributed their insights and experiences. Their willing assistance is greatly appreciated.

Project Methodology

The objective of this project was to gather insights from key stakeholders in the PBAC process, with special attention paid to HTA of complex innovative medicines including new oncology treatments. In order to gather this information, the following methodological approaches were employed:

Desk Research

Desk research was conducted to examine policies and processes of the PBAC along with several international HTA agencies, including: Canadian Agency for Drugs and Technology in Health (CADTH), pan-Canadian Oncology Drug Review (pCODR), the National Institute for Health and Care Excellence (NICE) and the Scottish Medicines Consortium (SMC). This included examination of their processes, tools and resources available publicly, via their websites. A review of recent literature was also conducted for abstracts or papers related to the processes, especially where relevant to patient and advocate participation[1].

Interviews

A series of in-depth interviews were conducted. The first group of interviews included experts in Australia (PBAC deputy chair, a prominent academic in this field, and representatives of several patient advocacy organisations).

The second group of interviews were with representatives of NICE, CADTH and other internationally recognised experts not aligned with a specific agency.

Where it was not possible to conduct an interview, information was also validated via email with additional agencies and some patient advocacy organisations in the above jurisdictions.

The interviews focused on what may be working well in terms of patient engagement, why that is the case, what barriers continue to exist, what resources are being applied and overall experiences.

Survey

An online survey was constructed in order to assess several key themes, including:

- General consumer understanding of the current PBAC process
- PBAC submission process relating to consumer engagement
- PBAC feedback process relating to consumer engagement
- Future possibilities for improving consumer engagement in HTA

The survey was circulated to Australian patient advocacy organisations, patients / cancer survivors and care givers to seek their understanding around these topics. The broader goal of the survey

was to help contextualise the feedback and insights gathered from the desk research and interviews.

Workshop

Finally, a workshop was initiated by BMS Australia bringing together patients, patient organisations and HTA professionals in order to present findings and seek feedback on the project work to date. A summary of the Australian HTA system, international HTA systems and visions for the future were shared and discussed with workshop delegates. The findings were subsequently collated, synthesised and extrapolated into the broader project in order to form the basis of the recommendations outlined in the final section of the report.

Current PBAC Process

The current PBAC review process is a multi-stage procedure that occurs over a 17-week period (**Fig. 2**). The assessment process begins with a written submission by the sponsor (usually a pharmaceutical company) to the PBAC. The presented evidence in the submission comprises clinical data to demonstrate the efficacy of the drug relative to an agreed comparator product on the PBS, as well as economic evaluation (often including complex modelling) to demonstrate cost-effectiveness (if a price higher than the comparator product is being sought). The submission is evaluated by an academic centre contracted to the Department of Health (DoH). The PBAC subsequently weighs the various forms of evidence contained within the submission, along with this expert evaluation, and presents a funding recommendation to the Minister of Health following March, July or November meetings. The company lodging the submission (the sponsor) may request a brief appearance at the PBAC meeting to address outstanding issues. The PBAC may decide to hold a hearing for consumers (patients and patient advocates) ahead of the meeting. For new cancer medicines, the Medical Oncology Group of Australia may be asked for comment ahead of the meeting also.

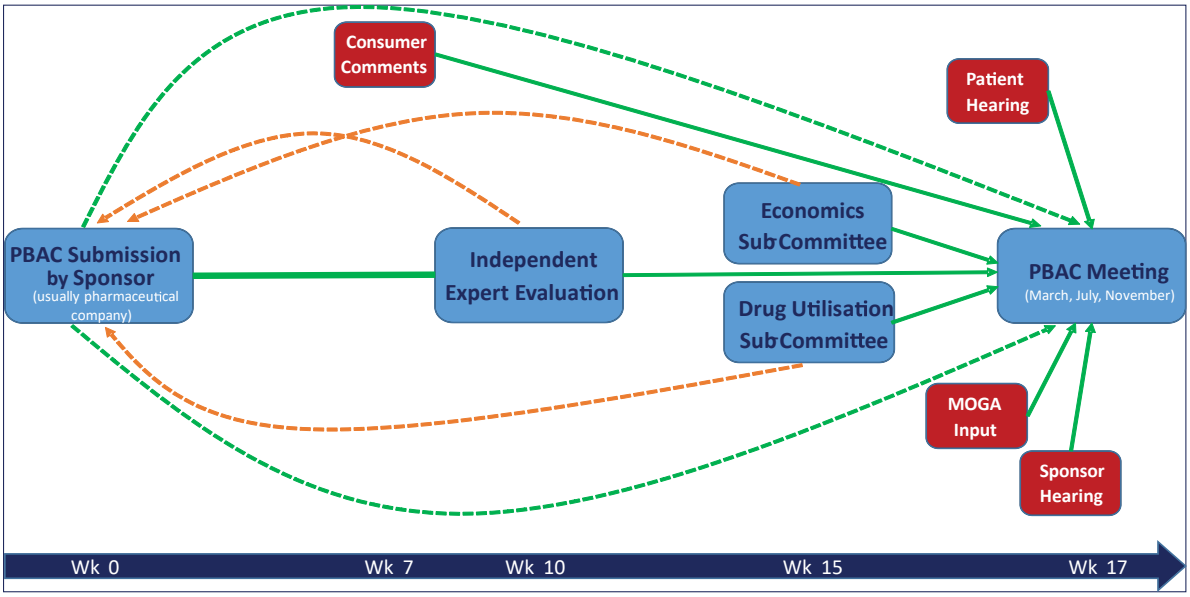


Figure 2: The 17-week PBAC process (central components: blue; additional components: red)

Evolution of Patient Engagement in Australia's HTA Processes

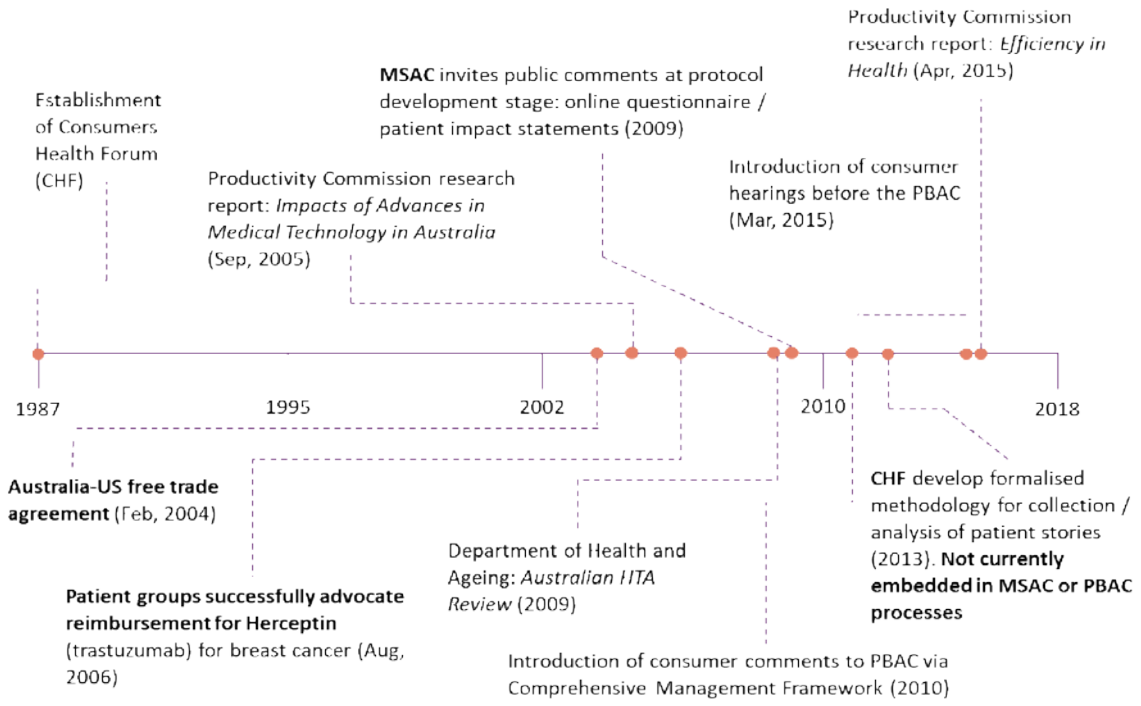


Figure 3: Key Milestones in Australian HTA[2]

Consumer Health Forum

In the 1986/87 Federal Budget, Government funds were allocated to establish the Consumer Health Forum (CHF). The CHF was established as a vehicle by which patient and community perspectives could be incorporated into a wider discussion of national health policy. Specifically, the CHF works to:

"…achieve safe, quality, timely healthcare for all Australians, supported by accessible health information and systems"

In 2015, the CHF recommended that PBAC appoint a second consumer representative, as one consumer representative is:

"…not consistent with the notion of active engagement or providing adequate support for consumer participation."

Consequently, the following benefits of having a second consumer representative have been realised:

- Allows more diversity of consumer experience and perspective with each consumer having access to their own networks and support mechanisms;
- Allows the representatives to specialise in particular areas, to work with patient groups in those areas and some provide more in-depth analysis and advice to PBAC; and
- Provides some pathway for succession planning as there can be a process of rotating the two positions so a new consumer representative has the support of an existing person

Australia-US Free Trade Agreement

Secured in February of 2004, the Australia-US free trade agreement (AUSFTA) has been associated with several improvements to the PBAC process. While these were not directly related to patient engagement per se, they did increase transparency of the process and open-up the participation of stakeholders (via the manufacturer hearings). Changes included:

- Manufacturing hearings before the PBAC
- Introduction of public summary documents (PSDs)
- Introduction of a review mechanism

Herceptin PBS Listing

The August 2006 addition of Herceptin (Trastuzumab) to the PBS represents an important milestone in the history of patient engagement in Australian HTA. In the late 1990s, the failure of Herceptin to meet cost-effectiveness criteria was thrice evoked by the PBAC as evidence for not including the drug on the PBS. Following sustained media attention and patient advocacy, PBS subsidisation of Herceptin was achieved in August 2006 via a special program outside of the PBS, lowering the cost of a weekly dose from AUD$1000 to A$30[2].

Australian HTA Review

In 2009, the Australian Government Department of Health and Ageing released a review of the Australian HTA system detailing the strengths and limitations of the current system (Australian Government Department of Health and Ageing)[3]. In response to the report, the Medical Services Advisory Committee (MSAC) introduced public consultation on protocols as well as patient impact statements in 2010. Moreover, MSAC processes were modified to invite public comments early in the assessment process (protocol development stage)[4].

Consumer Submissions and Consumer Hearings

The provision for consumers to make submissions related to products on an upcoming PBAC agenda was introduced in November 2008[5]. Consumer hearings were formally introduced into PBAC processes in March of 2015 in order to:

"…provide stakeholders with the opportunity for direct communication with the PBAC regarding medicines that are being considered for PBS listing[6]."

However, decisions to include a consumer hearing are somewhat ad hoc and patients / advocates receive very short notice to participate as well as limited guidance on how to prepare. Currently these hearings are held before the main PBAC meeting commences (on the day prior) although this is under review.

Additionally, an online resource for allowing consumers to provide written input on PBAC agenda items was also included and, over time, publication of the agenda was moved to 10-weeks prior to the PBAC meeting to allow 6 weeks for these inputs to be lodged. These elements were added with the view of consolidating consumer group feedback into the PBS assessment process.

Current Experience and Perceptions of Patient Engagement in Australia

Agenda and Submissions

PBAC submissions, generally from pharmaceutical companies, can be submitted at one of 3 times per year (*usually the first Wednesday in March, July and November*). A seventeen-week evaluation period is triggered upon submission, with the PBAC meeting at week 17 (*usually the first Wednesday to Friday in March, July and November*) to discuss and make a recommendation as to whether the application warrants PBS listing.

The PBAC agenda is made public 10 weeks prior to the committee meeting and based on submissions generally from pharmaceutical companies. Input from consumers is timed so that it is available to the PBAC consumer representatives to collate and summarise for the PBAC meeting.

Comments on agenda items are invited via PBAC & PBS websites (via an optional template) and presented to the PBAC by a consumer representative on the committee[7].

Whilst most consumers were aware of the ways in which they could participant in HTA decision making, our survey demonstrated mixed feelings regarding the utility of the currently provided template:

"There is a need for increased guidance as to what information PBAC needs from submissions... more feedback / transparency in general would be helpful"

"Consumers / patients generally receive no support on the submission process. The online form is complicated and difficult to follow"

"I thought the impetus came from patient groups? I was not aware of much support from the PBAC; I do know they have a support person, although, I don't know about their role"

"The form is difficult [to use] and it is not clear how to submit something beyond the standard template. For example, graphs or statistics cannot be imbedded in the submission template"

"Templates and links to all relevant documents would be very helpful. Consumers are doing this out of work time often in addition to work and family and treatment, or when unwell. The easier it is for them to become involved the better the information PBAC has to make its decision."

During interviews, some advocates reported high levels of workload (especially in the oncology area) where the number of new treatments being assessed by PBAC means organisations are often busy developing submissions / comments. It was suggested that the sheer volume of work made it extremely difficult to prioritise efforts to best serve patients. However, despite the acknowledged difficulties in prioritising efforts, there is a prevailing view amongst stakeholders that it is important to maintain the balance between input from organisations and from individual patients, as the latter sometimes provide the best insights.

Consumer Hearings

Interviews and survey data indicate that there is broad support for consumer hearings from consumer organisations as well as the PBAC. This addition to the PBAC process has evolved from what some described as initially quite an intimidating experience; into a very empowering one, with immense value derived from the process by both sides. However, decisions to hold a consumer hearing appear to be somewhat ad hoc and organisations who have participated have indicated that more guidance from the PBAC would be useful.

"We have had women present and believe the support is adequate"

"…although limited due to resource and time constraints [consumer hearings] are a great addition to the more passive advocacy submissions"

Multiple Submissions

The volume of new oncology drugs in development, in combination with the increasing complexity of clinical trials, has placed an increasing burden of work on both assessment agencies and sponsors. However, consumers have expressed frustration at the fact that multiple submissions are often required before a drug is approved and can view the failure of the initial submissions as being either 'errors' or 'miscommunication' on the part of the sponsor and/ or issues with interpretation of consumer comments. These comments suggest a lack of appreciation of the different perspectives of value that can exist between manufacturer, consumer and PBAC, as well as lack of understanding of the technical complexity and scope for 'uncertainty'.

Training and Education

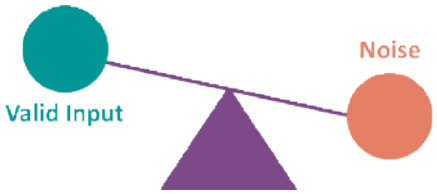
A frequently recurring concern expressed by consumers is lack of knowledge of HTA in general and the PBAC process in particular. Various efforts have occurred over time to address this, often supported by pharmaceutical companies, in part because of the lack of other options for consumers. In 2017, the PBAC initiated a consumer representative-facilitated workshop to educate interested parties into HTA in general, as well as Australian-specific processes (including how submissions are evaluated). Many participants found the workshop very helpful; however, some regarded the content as too technical. Experience from these educational outreach programs highlight the complexity and nuance required in order to

capture all relevant stakeholders' viewpoints and to strike the right balance of technical detail and lay-friendly language and terminology.

Consumer Representatives

Consumer representatives play a vital role in assisting individual consumers and consumer organisations in navigating the complex HTA process. Despite the broad recognition of value provided by consumer representatives, there is a perception that resource limitations have prevented consumers from accessing or utilising the full range of services potentially provided by this role. Moreover, consumers have expressed a need for more education around the specific functions provided by consumer representatives:

"Patients need further education on how to access (and submit to) consumer reps. Increase the number of consumer reps"



Conversely, stakeholders within the Department of Health have indicated that the recent deluge of submissions by consumers prior to PBAC meetings has made it extremely difficult to distinguish between 'noise' and 'valid input'. These 'lower quality' submissions indicate that further education into the decision criteria employed by the PBAC to assess new medicines would greatly benefit consumers and the Committee alike.

Feedback

Whilst consumer input (via submissions) is generally acknowledged in public summary documents, the lack of specific detail regarding the usefulness and effect of that input on the decision is a source of frustration amongst both individuals and consumer organisations.

"Currently, there are clear ways you can contribute through submissions, however, how effective your submission was you will never know as there is no feedback and little transparency"

A similar method of acknowledging consumer input is employed via consumer hearings, whereby a record of the hearing is made publicly available via the PBAC portal on the Department of Health's website. These documents are summarised by PBAC and provide a general description around the drug's indication, related consumer concerns (incl. physical, emotional and financial) and comparisons to analogous drugs previously listed or approved overseas. However, as with the public summary documents, consumers have expressed concern around the technical nature of the language in feedback documents.

Communications

Communication methods and channels are important in order to effectively engage (and accurately represent) consumers in the evaluation of new medicines. The PBAC's meeting agenda and call for consumer comments are published on the PBS website, with hyperlinks provided to the relevant submission forms for consumer input. Similarly, feedback related to decisions on new medicines is made available via the 'PBAC Outcomes' hyperlink on the website, as well as via public summary documents (also available on the website).

Individual consumer input has previously been acknowledged as potentially adding immense value to the consumer representatives during the evaluation process (and to the committee deliberations overall). However, due to resource limitations at the Department of Health, on-going and deeper engagement with consumers / consumer organisations generally occurs via personal relationships and on an ad hoc basis. Moreover, a general distrust of the pharmaceutical industry similarly acts as a barrier to good communication of information and joint dialogue between companies and consumers. Other stakeholders are sceptical of any engagement between companies and advocates, perhaps missing the value that genuine dialogue and information sharing might bring.

The insights obtained in this research is summarised into three categories in **Figure 3** below.

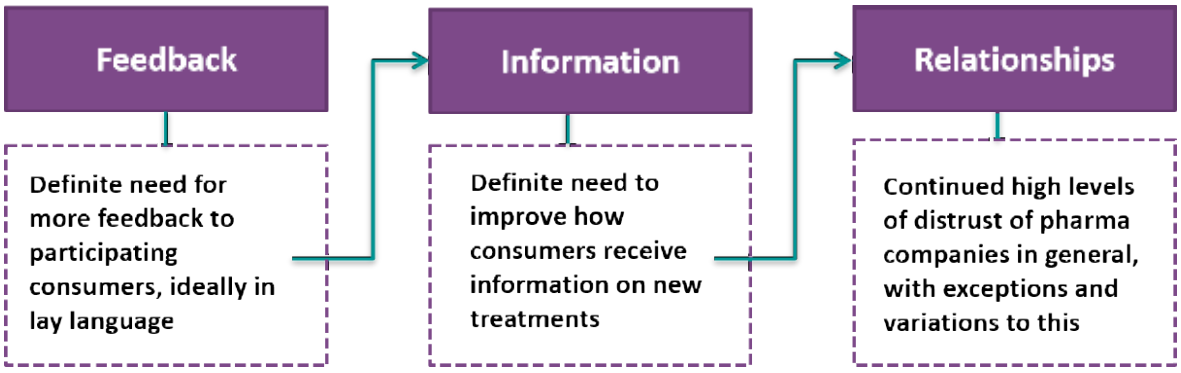


Figure 3: Important Themes Voiced by Consumers During Surveys & Interviews

Insights Into International Examples, Tools and Processes

The National Institute for Health and Care Excellence

The National Institute for Health and Care Excellence (NICE) has a specific directive to involve members of the public and patients in the appraisal of new medicines. The agency has a dedicated patient engagement support team, the Public Involvement Programme (PIP) that supports and advises patient involvement across NICE's entire portfolio. Moreover, NICE uniquely provides opportunities for patient engagement across all stages of appraisal and takes the view that "colloquial evidence complements scientific evidence".

Process	Patient Involvement
Scoping	• Patient organisations participate in consultation
Evidence Submission	• Evidence submitted by patient experts / patient organisations
Technical Consultation	• Added in 2018 to: consider the scientific and technical evidence submitted and arrive at preliminary scientific judgements
1st Committee Meeting	• Public meeting • Lay members present summary of patient issues / take part in decision making • Patient experts answer Qs & participate in discussion
Consultation	• Patient experts / patient orgs / public can comment
2nd Committee Meeting	• Public meeting – lay members take part in decision making
Final Recommendations	• Patient organisations may: comment on factual inaccuracies OR appeal on specific ground • Appeal meetings are publicly held
Review	• Patient organisations are included in consultation on whether guidance should be reviewed

Figure 4: Diagram of NICE's Review Process (modified from K. Facey et al. (eds)., Patient Involvement in Health Technology Assessment, 2017).

NICE are often regarded as exemplars of 'best-practice' for patient engagement in HTA, however, the deep integration of patient and public engagement within NICE also creates a challenging dynamic in which two, somewhat opposite views are expected to be simultaneously considered:

- Expert opinion from clinicians, health economists, researchers & NHS managers assessing clinical evidence and cost-effectiveness, and
- Patient 'voice' describing emotional and social impacts that may be addressed following a positive reimbursement recommendation

Moreover, although NICE provides formal structures and processes for patient engagement within in its appraisals, some researchers have suggested that:

"…the role of such groups is confined to the realm of 'representation' rather than that of a key stakeholder in decision making"

This perceived lack of alignment between the intentions behind patient involvement and the outcomes of their participation has led many advocates to believe that patient involvement is simply a 'box-ticking' exercise for many HTAs[8].

Citizens Council

The Citizens Council was established in 2002:

"…to ensure the perspective of the public is reflected in the methodology and processes that NICE uses to develop its guidance"

The Council is comprised of 30 members who meet in an open forum once per year for 2 days. Members are appointed via an independent organisation and meetings are run by independent facilitators. Post-meeting reports are made available for public comment and presented to NICE's board for discussion. Topics for discussion during the citizen's council meetings are selected during the guidance development process as a result of NICE's advisory bodies' activity.

Masterclasses

NICE's public Involvement Programme (PIP) runs a set of 'workshops' designed to inform patients / members of the public about NICE's activities and how to participate in NICE's work

- Participants are introduced to NICE's guidance, standards and advice
- The masterclasses also involve interactive exercises, designed to enhance participant knowledge and potentially facilitate involvement in processes

Patients Involved in NICE

The Patients Involved in NICE (PIN) initiative is a coalition of >80 patient organisations that meets four times per year and is:

"committed to enabling patient groups to engage productively with NICE"

This body works alongside PIP but is independent from both NICE and the pharmaceutical industry. There are also opportunities for patient groups to contribute to PIN's work via email or through related events.

Technical Report

The technical report is developed after the internal and external evaluation of the company's submission and includes:

- the company submission (and model when appropriate)
- the Evidence Review Group's critique of the company submission
- statements from stakeholder organisations and clinical and patient experts
- the overview of the discussions with the company about the technical aspects of the case
- preliminary scientific judgements of the technical team

A technical 'consultation' has been newly established in 2018, involving a team of NICE staff and the committee chair, which occurs before the appraisal committee meets to consider the scientific and technical evidence submitted in order to arrive at preliminary scientific judgements. The resulting technical report will be submitted to the appraisal committee for its consideration.

Table 2. Strength and Limitations of NICE's Patient Engagement Strategies as Informed by Desk Research, Interviews and a Recent workshop involving 20 Consumer Advocates in Australia

Strengths	Limitations

• Large organisational capacity to incorporate patient engagement
  • Long history of patient engagement in HTA
• Dedicated Citizen Council with 30 members recruited by an independent organisation
• Clearly outlined patient / public involvement policy
• Workshops and dedicated, independent bodies designed to inform / engage patients in their HTA processes
	• The large number of bodies involved in HTA could lead to overly bureaucratic system
	  • Extra governance demands / burden on patient organisations
	• Many functions to serve patients, but 'cost-effectiveness' reimbursement threshold many negate any input

The Canadian Agency for Drugs and Technology in Health

Canadian HTA occurs across multiple levels, including: hospital, regional, provincial / territorial and national. Patient organisations have been involved in CADTH HTA decisions since 2010.

Broadly, patient engagement in Canada can be categorised into the following areas: stakeholder feedback, synthesis of public literature, patient input templates, interview & focus groups and committee participation. CADTH formally solicits feedback from stakeholders (health care professionals, patients, drug manufacturers, associations, and other interested parties) on projects and draft reports via the 'provide input' tab on its website.

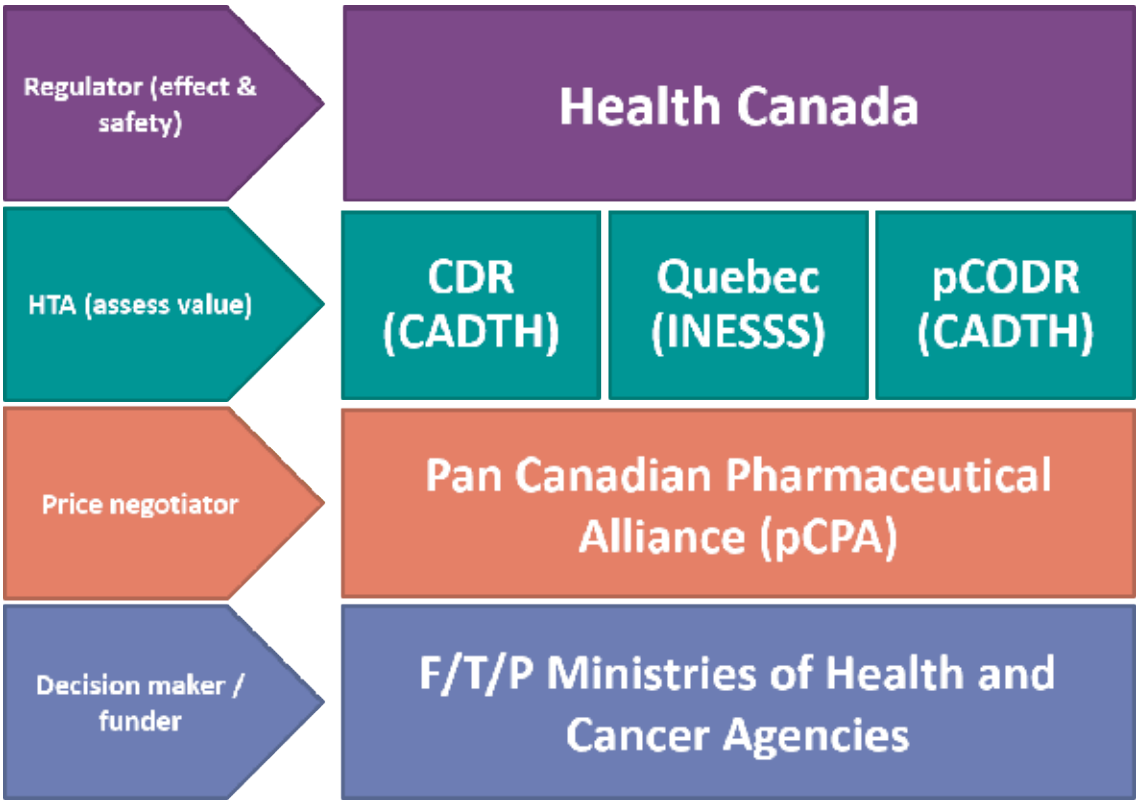


Figure 5: Overview of Canadian HTA System[9]

Canadian HTA has a separate pathway for assessing oncology medicine known as the Pan-Canadian Oncology Drug Review (pCODR). pCODR is a single-technology assessment programme operating under CADTH that undertakes 20-25 HTAs per year and considers evidence from several sources, including patient organisations, drug manufacturers, clinician-based tumour groups, and the pCODR Provincial Advisory Group.

The pCODR invites patient input at two points during the review process:

1. Early in the process for use in preparation of reports used by the pCODR Expert Review Committee (pERC) to develop its recommendations;
2. After pERC makes its initial recommendation*

*Input is provided via a template on the CADTH website; however, it is important to note that patient organisations/individual patients may only provide feedback on pERC's initial recommendation if they were involved in stage 1 of the review.

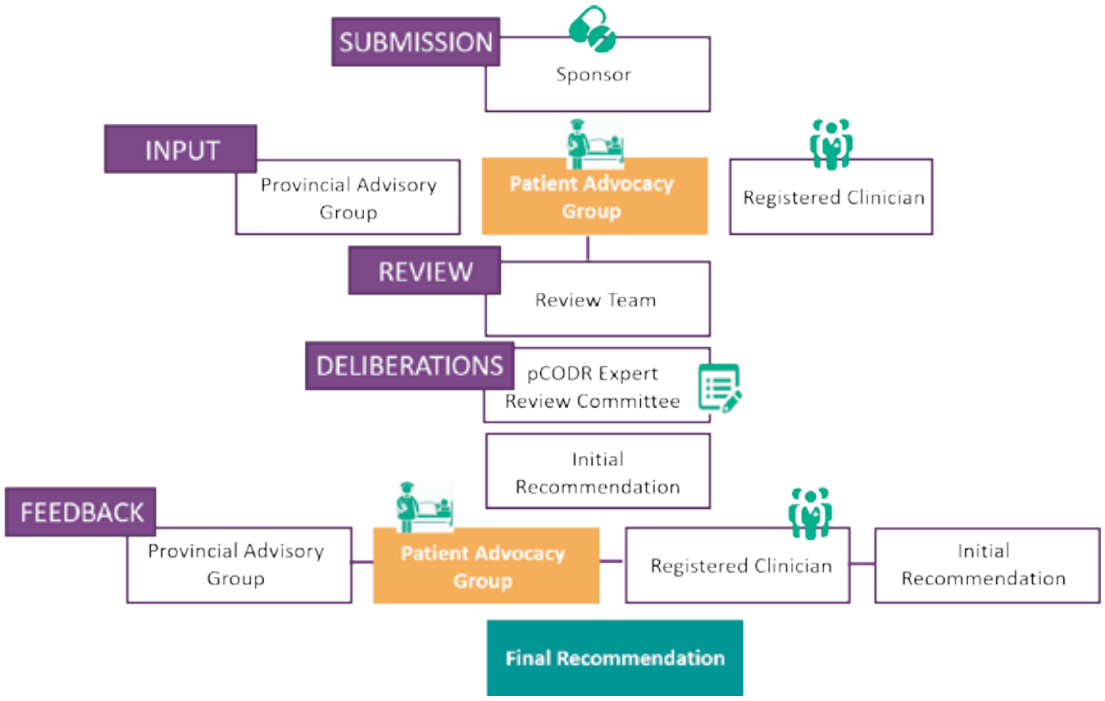


Figure 6: Patient Engagement in pCODR[10].

Submissions and Templates

Institut national d'excellence en santé et en services sociaux (INESSS) in Quebec, Canada was a pioneer in accepting patient input as part of its assessment process. In 2007, the Institute began accepting unstructured patient input via letters or emails. Other Canadian HTAs began accepting patient input in 2010 and subsequently standardised the process by developing a template.

From 2010 – 2015, CADTH's common drug review (CDR) has involved 114 patient organisations who have completed 297 patient input templates, contributing to 142 reimbursement decisions[11].

In early 2018, CADTH updated its online template in order to implement several key changes related to the stakeholder feedback, including:

- Clarity provided on what is needed to report information gathering

- Re-introduced questions on disease experience
- Focused questions on improved outcomes
- Simplified the required conflict of interest declarations
- Added a section for patient organisations to use when the drug under review has an associated companion diagnostic test

Feedback

CADTH's feedback process relating to submissions is relatively personal and detailed compared to other international HTAs. Once the review has been completed, CADTH writes back to each organisation or individual who submitted, highlighting the areas from their input that CADTH and its expert committee members found especially useful, and offering suggestions for future submissions.

Collaborative Workspaces

CADTH provides collaborative workspaces in which members of a pCODR patient group, clinicians, members of a tumour group and drug manufacturers or designated consultants are encouraged, "to submit and contribute drug review information, input, and/or feedback online." The formation of these work spaces is part of CADTH's broader goal to enhance access to high-quality information, knowledge, tools and resources for all stakeholders in HTA[12].

Digital Patient Engagement

CADTH provides a subscription-based service that alerts patients / patient organisations to the "*latest reports and recommendations, opportunities for input and feedback, and special events, including annual CADTH Symposium*."

This alert system can be tailored to the topic, frequency of communication and type of drug reimbursement recommendation (CDR / pCODR).

CADTH also provides Twitter updates calling for patient input on specific drug submissions that link to CDR reports with key milestones related to patient input.

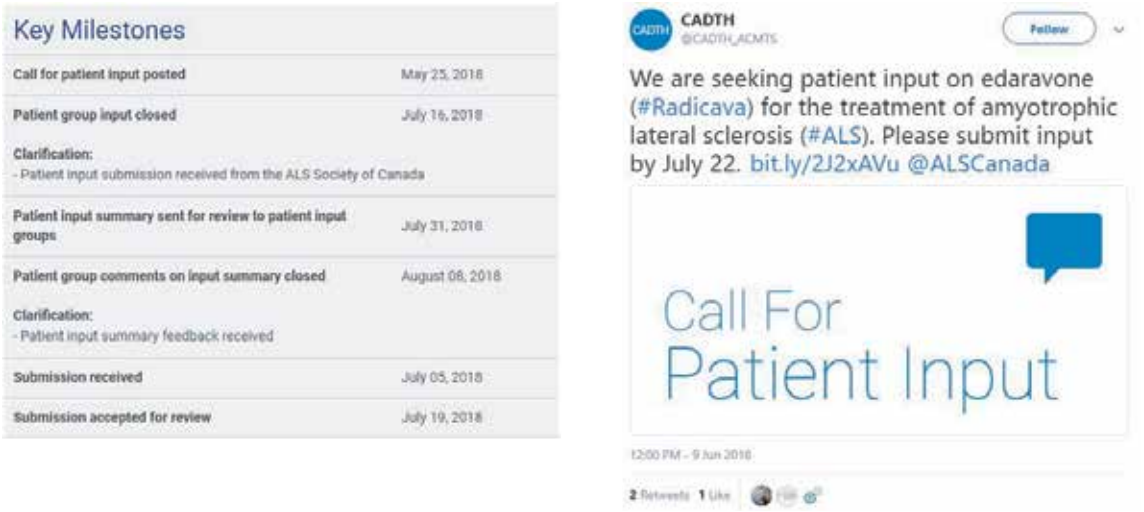


Figure 7: Example of an E-alert timeline and Tweet from CADTHs Twitter account calling for patient input

Patient Community Liaison Forum

The CADTH patient community liaison forum was established in 2013 in order to:

- Build understanding among members
- Identify priorities for patient engagement
- Facilitate the gathering of feedback on new patient engagement processes

The forum is staffed by a number of liaison officers, implementation support officers and program advisors who are located in provinces and territories across the country to provide better access to CADTH products and services for consumers.

Table 3. Strength and Limitations of CADTH's Patient Engagement Strategies as Informed by Desk Research, Interviews and a Recent workshop involving 20 Consumer Advocates in Australia

Strengths	Limitations
• Dedicated patient liaison officers help patients navigate the HTA process • Dedicated patient input page on website detailing timelines and mechanisms • Strong digital strategy / E-alerts for patient input with multiple channels utilised • Comprehensive, personalised feedback from submissions	• Patients / patient organisations must be registered with CDR in order to provide input • Perception that patients are not 'embedded' within CADTH's work • Lack of direct patient voice in expert review committee presentations • pCODR - Short timelines to develop and lodge submissions (~10 days)

Scottish Medicines Consortium

Patient involvement in HTA in Scotland was initially undertaken by the Health Technology Board for Scotland (HTBS). Like NICE, the HTBS developed mechanisms to encourage patient involvement throughout the HTA assessment process.

In 2001, the Scottish Medicines Council (SMC) was established with the goal of completing HTA of medicine within 12 weeks; however, rapid appraisal timelines limited the ability to include patient engagement. To alleviate this issue, the Patient and Public Involvement Group (PAPIG) was formed in 2002 and developed a structured template to help patients living with a condition to voice their experiences related a comparator or new medicine. In a process unique amongst global HTAs, PAPIG worked with the Pharmaceutical industry to develop the template in order to provide submitting patient organisations with information about the submitted drug.

Guides and Submissions

SMC's website has specific submission guidance for both pharmaceutical companies and patient organisations. Companies are provided with links to Patient Access Schemes (PAS) & Patient and Clinical Engagement (PACE)

Patient organisations are provided with the following overview guides;

- Guide for patient group partners (PDF)
- Preparing a submission for SMC - the patient group experience (Video)
- Patient Organisations submission example (ADHD) (PDF)

…and Submission Forms:

- Patient group partner registration form (DOCX)
- Patient group submission form (DOCX)

Summary Information for Patient Groups

In 2017, the SMC published a 'Guidance to manufacturers for completion of New Product Assessment Form' that detailed the necessity of including a Summary Information for Patient Groups (SIP) form with sponsor submissions to the SMC. This type of submission is valuable for the following reasons:

- Template is straightforward to complete
- Allows companies to explain to the indication choice to patient groups
- Presents an opportunity to more fully explain adverse events
- Enables translation of scientific evidence into meaningful language for patients and carers
- Provides patient groups with opportunity to request further information

- Provides a standardised approach to the information about the medicine needed to aid completion of patient group's own submission

PIN and PACE

The Public Involvement Network (PIN) was established in 2014 following a survey of 54 patient organisations noting the 'one-sided' nature of information flow (from patient organisations to the SMC).

- SMC now encourages patient organisations to register to become SMC Patient Group Partners
- Provides patient organisations with regular training days and a simplified patient organisation submission form

As of 2014, a Patient and Clinical Engagement (PACE) meeting may be requested by pharmaceutical companies following the rejection of orphan or end-of-life medicines (**Fig. 8**). In these meetings, discussion centres around the value of the medicine that may not be apparent in the clinical and economic evidence.

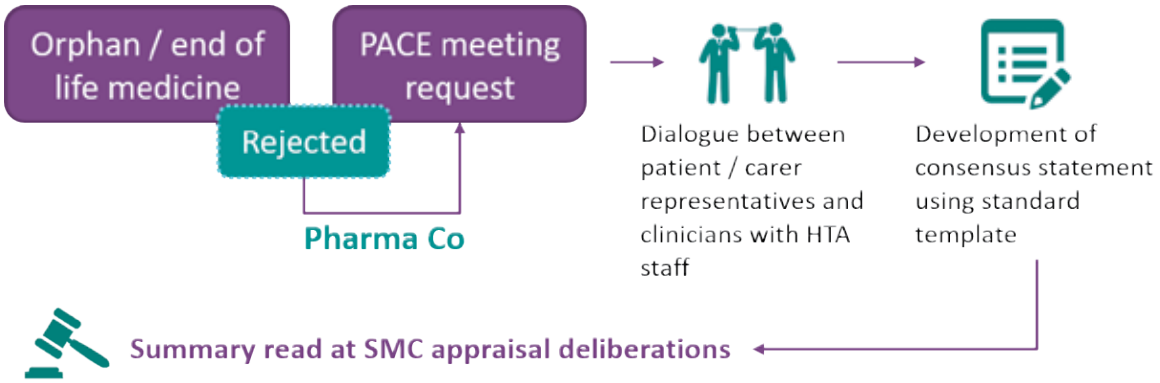


Figure 8: Overview of PACE meeting request process

Table 4. Strength and Limitations of SMC's Patient Engagement Strategies as Informed by Desk Research, Interviews and a Recent workshop involving 20 Consumer Advocates in Australia

Strengths	Limitations
• Long history of patient engagement in HTA • Website is easy to navigate / intuitive • Multiple guidance documents provided • Guidance / systems for patient input from both patient groups and industry	• SMC staff members present on behalf of patients • Focus deemed to be too heavily weighted towards patient advocacy groups • Patient voice may be neglected in this scenario

• Three dedicated functions for patient input • Public involvement officers dedicated to helping patients engage with SMC process and improving the focus of patient inputs to more specifically address issues of concern	• Criticism: nature of relationship between SMC / patient groups is 'one-sided' • Some pharma companies are hesitant to engage with PAPIG due to compliance concerns

Table 5: Comparison of International HTA Patient Engagement Mechanisms[12]

Organisation, Country	Stated reasons for patient / public initiatives	Mechanisms for patient involvement	Type of evaluations conducted; frequency of evaluation
NICE 		• Participation on a working group/committee • Identifying topics • Refining scope • Appraising evidence • Marking recommendations • Guidance on moral/ethical issues	• Participant satisfaction • Every HTA
CADTH 		• Participation on a working group/committee • Refining scope • Identifying clinical outcomes • Data collection • Reviewing reports • Disseminating results	• Process, impact • > 3 years
SMC 		• Participation on a working group/committee • Writing/reviewing reports • Appraising evidence • Making recommendations • Disseminating results	• Process, impact, participant satisfaction • Evaluation as a continuous improvement exercise

 Promote capacity building

 Enhance patient/public understanding of HTA

 Promote fairness & inclusion of a range of stakeholders

 Enhance patient/public support of decisions

 Help ensure decisions reflect patients/public values

Challenges and barriers

Australia has had a leading role in the application of HTA for medicines reimbursement and to some extent has been one of the countries of interest for others aspiring to implement this value determination approach. While the approach to patient engagement has evolved over time, there are opportunities to further improve and to learn from other successful systems and processes. It is tempting to envisage a 'gold standard' HTA process to aspire to and to consider the most mature and developed HTA systems as the model to emulate. However, the interviews with international experts in the area advised that in fact, HTA systems and processes need to reflect the context in which they operate. This includes national legislative frameworks, health policy, culture and overall health system resources.

That said, it is clear from the insights gained across a wide range of stakeholders that there are commonly recurring challenges and barriers to optimal participation of patients and advocates in HTA for medicines. While these are not unique to Australia, insights from local stakeholders across all components of this project reveal that all the following are areas of concern:

Education and training

In order for patients and patient advocates to engage effectively in an HTA process, a level of understanding is required regarding the overall aims and objectives of that process. This includes:

- an understanding of the legislative underpinnings, which are critical in defining what the process can and cannot consider when making recommendations.
- an understanding of the way in which the information (on which the recommendation will be made) is prepared and delivered to the HTA process.
- some understanding of the nature of the technical assessment that is conducted in the lead up to the appraisal committee process. While this step is more technical and complex than most patients / advocates require, some understanding is helpful in relating to the outcomes of the HTA process.
- understanding how the process provides opportunities for patient / advocate engagement and expectations of the PBAC regarding that engagement
- feedback can also be considered a key part of learning and education in any process. It is clear from advice received from advocates and patients that more feedback on any efforts to engage with the PBAC process would be appreciated – see the discussion below.

It is noteworthy that the availability and delivery of education and training resources in Australia has historically been fragmented and inconsistent. This partly reflects a lack of consistent application of resources within the Department of Health and PBAC for education and training purposes. Industry has, at times, attempted to fill that gap via funding of events and programs that provide education and training, sometimes based on internationally recognised models such as the London School of Economics program in this area or utilising visiting international experts.

Communication and feedback

As with any complex process, the communication of objectives, logistics, timing of key events / activities within that process and the touch points for external input are critical to understanding the process and the quality and quantity of the external input.

Lack of clarity of, or accessibility to, such information creates further barriers to effective and meaningful input from patients and advocates. While progress has been made in Australia, insights gained from a range of stakeholders suggests that there are limitations to the level and type of communications employed across the PBAC process. These limitations include:

- **Information available to consumer regarding medicines the PBAC will consider at any particular meeting:**

  While progress has been made by publishing the PBAC meeting agenda earlier (10 weeks prior to PBAC meeting vs previous 6 weeks prior to PBAC meeting) - to provide more time for consumer submissions, there continues to be a lack of information available to stakeholders about the medicines, the claims being made to support the value proposition and the type of evidence being presented by the manufacturer. In Australia this appears to be made more difficult by the Medicines Australia Code of Conduct and how it (and therefore its member companies) interpret the regulations related to communication of information on prescription medicines to anyone other than a registered medical practitioner. In addition, company compliance policies, which are not consistent between companies, can add further restrictions on what and how companies can communicate to patients and patient organisations during the HTA process.

- **Knowledge that a particular medicine is on the PBAC agenda:**

  At present this occurs via the release of the agenda 10 weeks prior to each PBAC meeting (when it is published on the PBAC website). However, for less common conditions without a well-resourced and experienced advocacy organisation, it cannot be guaranteed that the agenda will be reviewed (or even seen) in a timely way for every PBAC meeting. While this information could also reach patient organisations directly from the pharmaceutical company, that is not seen as the most appropriate avenue by some stakeholders.

- **Making submissions to the PBAC:**

  The PBAC website includes a template 'Online Comments to the Pharmaceutical Benefits Advisory Committee'. Insights from local stakeholders reveal that this resource is not easily found on the website, is not easily completed by individual patients and not flexible enough to accommodate the inputs from more sophisticated advocacy organisations. The PBAC also accommodates submissions (including simple letters) from individual patients (although this is not actually stated in the guidelines for patient engagement). This is a positive element welcomed by patients and advocates but in the absence of clearer guidance on what is actually considered as useful information stakeholders find it challenging to do this. An adverse consequence of the lack of guidance and early notification of the opportunity to write a letter is that sometimes 'form letters' become

the default option, resulting in larger numbers of similar letters that do not contain information of value to the PBAC in their decision-making.

- **Understanding PBAC recommendations:**

  The advent of the Public Summary Document (PSD) greatly increased the accessibility and clarity of information regarding recommendations made by the PBAC and the reasons for those recommendations. Over time the PSDs have evolved to be modified versions of the full PBAC minutes and are published approximately four months after each PBAC meeting. The PBAC acknowledges that the PSDs contain technical terms and information and refer readers to the Glossary available on the PBAC website. However, local stakeholders continue to find the information too complex, not sufficiently 'lay friendly' and not especially helpful in providing direction for how consumers can provide further constructive input that might contribute to shifting a rejection or deferral to a positive recommendation at subsequent PBAC meetings.

- **Feedback:**

  As noted, there are various ways in which patients and advocates can provide input to the PBAC process. However, the availability of feedback to stakeholders who have provided feedback on any specific medicine is variable. Where time permits, the consumer representatives on the PBAC may reach out to patients and advocates and discuss how their input was received. Local stakeholders indicate that this is more likely to happen for complex medicines or conditions where an ongoing relationship has been established with a leading advocacy organisation. The 17-week cycle that operates (from manufacturer submission to PBAC meeting) means that the consumer representatives are extremely busy and it is difficult to provide this level of feedback to all contributors. This in turn results in patients and advocates being unclear about what was useful (or not) in their input or how it might be improved for subsequent medicine submissions.

Resources

It is apparent from the insights obtained that many of the current barriers and challenges to more effective patient and advocate participation are related to the level of resources available. Improved communication, increased education / training, enhanced opportunities for engagement and increased understanding of PBAC recommendations are all somewhat dependent on adequate resources within the Department of Health in support of the PBAC processes. The current consumer representatives on the PBAC are held in high regard by the advocacy community and individual patients who get the opportunity to interact with them. However, the nature of the 17-week PBAC cycle plus the fact that the individuals are also full members of the PBAC combine to mean that they are constrained in the time they have available to address any of the above-mentioned barriers.

Early engagement and horizon scanning

A recurring theme in local stakeholders' insights is that they regard the whole PBAC process as very constrained in time and not conducive to thoughtful input. This is particularly so with the more

experienced advocates; the more the understanding of the PBAC needs and processes, the more the realisation that with increased time, one could develop more constructive input. While not necessarily able to articulate this need in technical terms, it appears that most experienced advocates would welcome opportunities for earlier engagement on new medicines, ahead of a submission by the manufacturer that initiates the actual PBAC process. It is also understood by some of these stakeholders that 'early engagement' (whatever form that might take) may not be possible (nor necessary) for all new medicines and that some form of prioritisation may be useful if early engagement options were to become a reality.

There is some awareness of the concept of horizon scanning, with a few of the more experienced advocacy organisations already conducting some form of this. Others lack knowledge of the concept but do see the value of early awareness if there was a mechanism to achieve this.

However, some advocacy organisations involved in a disease or condition with a continuous stream of innovation (e.g. some cancers), indicated that they experience a considerable workload and challenge in keeping up with each PBAC agenda and developing and making submissions to the PBAC. Capacity and workload may be constraints regarding both additional early engagement and horizon scanning and would need to be considered if further opportunities for these were to be created.

Recommendations

This report makes 9 recommendations for consideration (**Table 6**). As with any complex process involving many varied stakeholders, some of these recommendations are easier and more straightforward to implement that others. Some would require longer time frames and extensive consultation with the range of stakeholders. Some may be suited to a pilot approach.

Table 6. Summary of 9 Key Patient Engagement Recommendations and Associated Difficulty of Implementation

Recommendations	Difficulty Implementing
1. The use of e-alerts to advise interested stakeholders of a product entering the PBAC process	Easier / short-term
2. Prompts for submission deadlines	
3. Feedback on patient submissions	
4. Consumer-friendly public summary documents	Medium / longer term
5. Master classes in HTA and PBAC processes	
6. Valuation of evolving cancer survival outcomes	
7. Information on the products, provided to advocates by the manufacturer or via an independent third party	Hardest / pilot approach
8. Inclusion of advocates in a technical consultation prior to the PBAC meeting	
9. Horizon scanning	

For this reason, the report uses a matrix approach, where the recommendations are grouped according to extent of the potential reward or impact versus degree of difficult in implementation (**Fig. 9**).

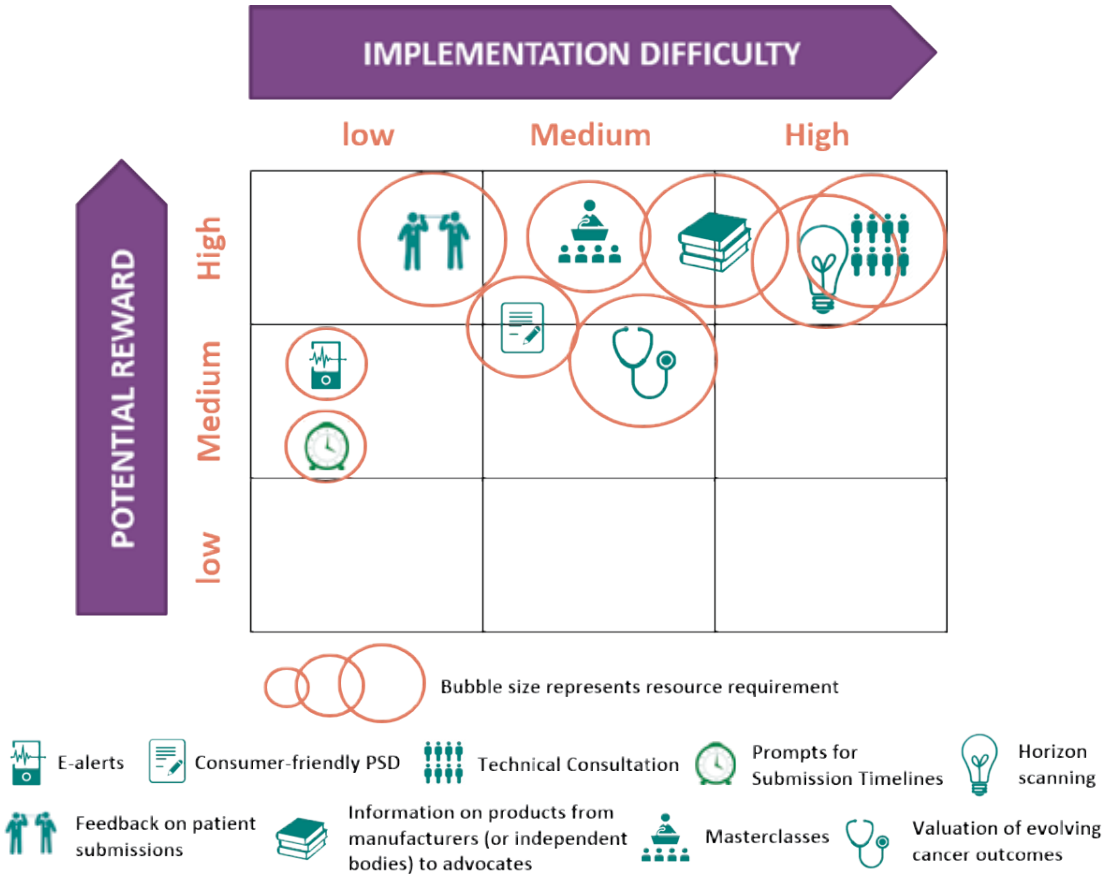


Figure 9: Matrix Representation of Recommendations for Patient Engagement in HTA

Recommendations feasible for early implementation (Easier/ Short-term)

There are several recommendations related to the introduction of aids to communication of PBAC processes. It is important to note that while the recommendations in this category are relatively straightforward to implement, they do require an increase in the human resources available at the Department of Health / PBAC to support consumer engagement. While the current consumer representatives on the PBAC do an excellent job, they are also full members of the committee and therefore lack the time to deliver on communication improvements. Comparisons with international peer organisations reveal that while the number of people dedicated to supporting patient or consumer engagement is not necessarily larger than with PBAC, the difference is there are people employed within the relevant department or organisation solely to support these activities. These people do NOT have additional and demanding roles on the decision-making committees themselves.

Therefore, it is noted that in order to successfully implement these recommendations that there is an increase in human resources, specifically the appointment of consumer engagement support staff at the Department of Health.

The recommendations in this category are:

Recommendation 1: The use of e-alerts to advise interested stakeholders of a product entering into the PBAC process:

This has been successfully implemented in Canada (described earlier in this report). There are several options for how his recommendation could be implemented in Australia. Patient advocacy organisations could be encouraged to register their interest in a therapeutic area with the Department of Health. An e-alert would then be sent to all parties who have registered such an interest. There are at least two options to consider: Option A is to send an alert when a submission is lodged with the PBAC process. Option B is to send the alert when the PBAC agenda is first made public (effectively in line with the current timing for consumers to become aware of manufacturers submissions to the PBAC). Option B would be the simplest as it would not require any additional stakeholder agreement but would simply increase the likelihood of advocacy organisations being aware of the product under consideration and therefore their ability to make a submission. Option A would be more challenging to achieve as it would require agreement from Medicines Australia. It would effectively make the act of a submission by a manufacturer public knowledge. While earlier awareness by advocacy organisations would undoubtedly increase the quality of the input, industry may have concerns regarding commercial competitiveness. This may be less of an issue if AMWG streamlined pathways work proceeds and leads to the removal of the current Minor submission deadline (*April, August, December*), which is after the deadline for Major submissions (*March, July, November*).

Recommendation 2: Prompts for submission deadlines:

Once advocates are made aware of a product on the upcoming PBAC agenda there is a narrow window for them to develop and lodge a submission. As above, by registering with the Department of Health, advocacy organisations could become eligible to receive electronic / digital prompts that would remind them of the upcoming deadline. This would help support advocacy organisations as they manage their submission process.

Recommendation 3: Feedback on patient submissions:

One of the frustrations most often and most strongly noted by consumers in Australia is the lack of feedback received following a submission (or otherwise engage in the PBAC process). This is not to say feedback does not occur. When the consumer representatives on the PBAC can meet directly with advocacy organisations about a specific product and associated PBAC recommendation, they are able to deliver clear and useful feedback. However, this is limited by both time and the extent of personal relationships between the consumer representatives on the PBAC and advocacy organisations.

Several of the international HTA agencies have developed processes for systematic feedback to consumers. For example, CADTH (in Canada) has developed a feedback letter template with three components: what was useful about their submission, how it aided the committee's decision / recommendation, and suggestions for improvement in future submissions.

It is noted that in order to successfully implement the recommendation that there is a need to increase human resources, specifically the appointment of consumer engagement support staff at the Department of Health.

Medium difficulty but high benefit recommendations (Medium/Longer-term)

Recommendation 4: Consumer-friendly public summary documents

The development of the PSDs as an outcome of the US-Australia Free Trade Agreement in 2006 was a big step forward in transparency of the outcomes of the PBAC process along with increased information on the rationale for those outcomes. For the first time all stakeholders were able to read a comprehensive account of the information considered by the PBAC, the general areas of critique of the manufacturer's dossier and the overall thought process of the PBAC in reaching their conclusion on a specific medicine. Over time, the PSDs evolved to be closer to the actual minutes of the PBAC meeting.

It is important to note that the PSD is in effect an account of the HTA process as agreed between the Department of Health and the manufacturer. The PSDs continue to employ ranges to report various quantitative aspects of the HTA review, such as a band within which the incremental cost-effectiveness ratio (ICER) for the product falls, rather than the specific ICER figure. Similarly, a band is used to describe the net budget impact for the medicine under review.

The feedback gained from the interviews, survey and workshop indicates that Australian consumers would appreciate and benefit from a version of the PSD which is less technical but still conveys the essence of the HTA review, the PBAC recommendation and the rationale for that recommendation.

Development of a 'consumer friendly' version of the PSD would require additional resources to develop a parallel document for each product undergoing PBAC review. Other stakeholders (notably the manufacturer) would need to review and agree to the 'consumer' version of the PSD in a timely way and issues regarding commercial in confidence information would need to be considered.

It is noted that in order to successfully implement the recommendation that there is an increase in human resources, specifically the appointment of consumer engagement support staff at the Department of Health.

Recommendation 5: Master classes in HTA and PBAC processes

Insights from the interviews, survey and workshop strongly support a call for increased availability of education and training on HTA for consumers. Such education needs to cover both the fundamentals of HTA as well as the PBAC processes. Examples can be found in several of the international HTA agencies and are described in this report. Feedback indicates that training should be offered annually at a minimum.

Over the past decade, several attempts have been made to deliver this type of education in Australia, often utilising funding from industry along with visits by international expert patient advocates.

More recently, there have been examples of such education delivered by the consumer representatives on the PBAC. This has the obvious advantage of independence from industry funding. However, it has been relatively infrequent and limited in reach, presumably because of the combined lack of additional resources in the Department of Health and the highly demanding role played by that individual as a full member of the PBAC.

It is noted that in order to successfully establish a 'masterclass' for consumers, that there is a need to increase in human resources, specifically the appointment of consumer engagement support staff at the Department of Health.

Recommendation 6: Valuation of evolving cancer survival outcomes

It is apparent that the new generation of cancer medicines are changing the way many patients experience their disease and the associated treatment. While survival gains are not always uniform across all types of cancer and all patients, there is evidence that some patients experience longer overall survival and / or longer progression free survival compared to older medicines.

In addition, the toxicities associated with some of the newer cancer medicines are very different to standard chemotherapy options. Experiences with toxicity are variable but overall suggest that estimating the health states experienced by these patients requires further consideration. In HTA, a patient's experience of quality of life during and after treatment is incorporated via assessments of what economists refer to as health state utilities. These can be quantified using various methods including questionnaires or other tools. When assessing these new cancer medicines, application of estimates of health state utilities from studies of conventional chemotherapy may not be relevant. If health state utilities are measured within trials of these newer medicines, the timing of these measurements and the way that these utility values are incorporated into the economic models also needs careful consideration. While this is a highly technical area, input from patients on actual experiences and how they differ across phases of treatment may be an important part of developing new economic evaluation approaches.

This is relevant to the discussion of patient engagement in HTA of cancer medicines specifically, as patients can provide the best insights into how periods of increased survival and reduced toxicity impact experience and quality of life.

As such, it is recommended that work is undertaken to determine more appropriate approaches to assessing the quality of life (including variability in health state utilities) for new cancer medicines. This should include a stakeholder meeting or workshop to include broad patient and care giver representation along with expert clinicians and health economists, to discuss issues with the measurement of health state utilities and incorporation into economic models.

It is noted that successfully implementing this recommendation, including delivering this type of stakeholder workshop, requires fitting it into a very busy landscape of meetings and PBAC cycles. Industry may be willing to discuss how this might be accommodated.

High difficulty but high benefit recommendations (Hardest/Pilot approach)

Recommendation 7: Information on the product, provided to advocates by the manufacturer or via an independent third party

Insights consistently indicate that (consumers feel inadequately informed to be able to contribute effectively to the PBAC process. Apart from the need to understand more about HTA in general and the PBAC process, there is also a desire to understand more about the medicines under review.

An excellent precedent exists in Scotland, where a template is provided by the HTA agency (the Scottish Medicines Consortium) to the manufacturer. The manufacturer completes the template which is then made available by the SMC to interested consumers.

There is the potential to adapt this in Australia. The issue is that the regulations related to provision of information on prescription medicines to patients are interpreted by the Medicines Australia Code of Conduct in a limited way. Presumably this is to avoid any impression that companies are in breach of the regulations and might be trying to influence patients to request a specific medicine rather than the medicine being selected by their treating healthcare practitioner. Individual companies who are members of Medicines Australia may also differ in their adherence to the Code of Conduct, with their international compliance requirements often contributing a further complication.

The use of a template that is controlled by the Department of Health and completed by the manufacturer (subject to approval by the DoH) may be the best way to resolve this complex issue. However, it is unlikely to be implemented quickly as it would require agreement from Medicines Australia and their member companies. It would also be important to consider any implications from a regulatory perspective, especially regarding the current regulations relating to communication of information on prescription medicines.

There are precedents for the provision of information within the PBAC process via an independent third party. For example, the Medical Oncology Group of Australia provides summary information to the PBAC on the place of new oncology medicines in clinical practice. This example is of course a more technical exercise, with the PBAC as the recipient of the information. This is not the same as providing such information to consumers and consumer organisations, where the information would need to be presented in lay terms. However, it does demonstrate that it is feasible an independent third party could deliver this function and provide a summary of a new product about to be considered by the PBAC, in ways that would be helpful to the preparation of consumer comments.

Recommendation 8: Inclusion of advocates in a technical consultation prior to the PBAC meeting

When a product is submitted by the manufacturer to the PBAC the dossier undergoes extensive technical review by a contracted academic centre. That review is provided to the manufacturer for comment before going to the PBAC subcommittees and eventually to the PBAC itself (where a summary of the review and the manufacturer response are considered).

In England, NICE has implemented a step where the equivalent technical review is discussed in a stakeholder meeting, before the summary is considered at the equivalent of the PBAC (the NICE appraisal committee). This step has been termed the 'technical consultation'.

Initially, that consultation included the NICE technical team responsible for the product in question and the manufacturer. Representatives from the evidence review group who conducted the technical review may also be present.

More recently, it has been expanded to include patient advocates and expert clinicians. The main objective of this is to identify the key issues that the upcoming appraisal committee will need to consider in reaching its decision / recommendation. It is believed that including patient advocates in this discussion may help bring their perspective into the consideration of those key issues, so that by the time the product is considered by the appraisal committee, there are well-developed perspectives that will help them in their deliberations.

This step would not be simple to introduce into the PBAC process. It would potentially disrupt the current 17-week cycle (from submission to PBAC meeting). However, a mechanism to increase patient engagement for medicines considered 'high added therapeutic value' could be introduced under the proposed framework currently being considered by the AMWG streamlined pathways subgroup.

Consultation would also be required with Medicines Australia and its member companies, as the step would mean a wider dissemination of the technical issues before the PBAC meeting, as compared to the current release of information post-hoc in the form of the PSD.

Recommendation 9: Horizon scanning

Several stakeholders in Australia raised the issue of wanting to have increased early awareness of new medicines. There are obvious benefits in terms of more time to think about implications, potential differences between the new medicine and existing options and how best to develop patient-focused evidence. More time would potentially allow advocates to plan and implement surveys or other approaches (such as workshops, discrete choice experiments), ultimately leading to more useful and broader evidence that could be delivered through existing and/or enhanced patient engagement channels.

This type of activity is broadly termed horizon scanning and has been the subject of much discussion in HTA circles in recent years. Currently, various jurisdictions do it to varying extents and with varying resources (for example NHS England and pan-European efforts).

However, in the context of this report, the recommendation is more specific to the advocacy community and could even be considered specific to the oncology advocacy

community. Horizon scanning of potentially significant and complex new medicines (or medicines for complex diseases with high unmet need) could be commissioned specifically to inform the advocacy community.

There are many challenges to be considered in this regard. Not least is funding, along with who would conduct the horizon scanning, how would medicines be identified and prioritised, and how would the reports be accessed, noting the importance of balancing technical accuracy with lay language and usability.

It is recommended that a planning group be established to consider the potential for horizon scanning to inform patient advocacy in Australia. If necessary, this effort could be focused on the oncology area in the first instance, to pilot a process which could be further expanded if successful. While the challenge of finding appropriate time and resources to undertake this puts it in the 'high difficulty' category, it is likely to bring substantial benefit in terms of higher value patient engagement and evidence.

Conclusions

Australia has been prominent in the use of health technology assessment to support decisions on reimbursement of pharmaceuticals since 1993. Over time, the recognition of the societal importance of consumer input to that process has grown, along with the introduction of mechanisms for patients and patient organisations to engage.

It is clear from the insights gained in this project from patient organisations, individual patients as well as HTA decision makers - both within Australia and Internationally - that there is potential to improve upon the Australian system.

Recommendations within this report range in implementation difficulty but there are some excellent practices and tools in use in other jurisdictions that can be drawn upon to enhance the Australian system and add value

With good stakeholder involvement and a commitment to applying more resources in this area, there is the prospect of Australia again being a leader in this important aspect of HTA.

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