Recommendations to improve the patient voice in health technology assessment in Australia

Patient Voice Initiative Incorporated (NSW Association)

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## Abbreviations

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<tr>
<th>Abbreviation</th>
<th>Full Form</th>
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<tr>
<td>AGENAS</td>
<td>National Agency for Regional Health Services (Italy)</td>
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<tr>
<td>CADTH</td>
<td>Canadian Agency for Drugs and Technologies in Health (Canada)</td>
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<td>G-BA</td>
<td>Federal Joint Committee (Germany)</td>
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<td>HTAi</td>
<td>Health Technology Assessment international</td>
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<td>IQWiG</td>
<td>Institute for Quality and Efficiency in Healthcare (Germany)</td>
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<td>MSAC</td>
<td>Medical Services Advisory Committee (Australia)</td>
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<td>NHIA</td>
<td>National Health Insurance Administration (Taiwan)</td>
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<td>NICE</td>
<td>National Institute for Health and Care Excellence (England and Wales)</td>
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<td>PBAC</td>
<td>Pharmaceutical Benefits Advisory Committee (Australia)</td>
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<td>PLAC</td>
<td>Prostheses List Advisory Committee (Australia)</td>
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<td>SMC</td>
<td>Scottish Medicines Consortium (Scotland)</td>
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Purpose of this document

This document makes recommendations for improving consumer involvement in health technology assessments conducted by MSAC, PBAC and PLAC. These recommendations are based on the needs expressed by patients and patient groups at workshops convened by the Patient Voice Initiative. This document contains suggestions for how these needs can be met and examples of how other HTA bodies do it.
**Acknowledgements**

The strength of the Patient Voice Initiative to date has been the collaborative nature of the events. PVI gratefully acknowledges the involvement and enthusiasm of a wide range of stakeholders and importantly, their recognition of the importance of the work of the initiative.

We are indebted to the patients and patient groups representing a wide range of diseases and conditions who generously offered their experience, time and networks.

PVI would like to thank Geoff Nyssen, who co-chaired the Melbourne meeting, and all the patients who spoke at the events across Australia, sharing in depth your stories and experiences. We would also like to thank Richard and Kate Vines and their team at Rare Cancers Australia who were extremely generous in their contribution of time and resources at all of the events.

To the past and present members of the PVI steering committee and those involved in compiling this report including Dr Kim Hamrosi (CaPPRe), thank you for your commitment to this important work. Many of you have gone above and beyond expectation contributing time and experience to ensure the success and continuation of the initiative and its events.

For a comprehensive list of our steering committee (past and present) and valued PVI sponsors, please refer to the back of this document.
Foreword

In 2015, a group of stakeholders from industry, academia and patient groups came together to discuss methodologies and approaches for eliciting the patient perspective on the value of medicines. As a result of these meetings, a conference called Room with a Patient View was organised with the aim of increasing patient engagement in health technology assessment (HTA) in Australia. Following the conference, a steering committee was formed to action items generated from the workshops.

In 2016, the group became known as the Patient Voice Initiative to reflect the desire and motivation of stakeholders to grow the presence of the patient voice in HTA used by Australian bodies such as the Pharmaceutical Benefits Advisory Committee (PBAC), Medical Services Advisory Committee (MSAC) and Prostheses List Advisory Committee (PLAC) for the reimbursement of new drugs, devices, procedures and prostheses.

The Patient Voice Initiative formally registered as an Incorporated Association with the NSW Government in March 2018.

To date, the Patient Voice Initiative has held events in Sydney, Melbourne, Brisbane and Canberra which have seen patients, patient advocacy groups, industry, academia and government working together towards a common goal.

We adopt the Health Technology Assessment International (HTAi) definition of HTA:

“HTA is a multidisciplinary field that addresses the clinical, economic, organisational, social, legal and ethical impacts of health technology considering its specific healthcare context as well as available alternatives. The scope and methods of HTA may be adapted to the needs of a particular health system, but HTA processes and methodologies should always be transparent, systematic and rigorous. In health systems throughout the world, HTA plays an essential role in supporting decision-making.”

Patient Voice Initiative Mission

- Deliver a forum for collaboration and dialogue within a public-private partnership that encourages broad participation of a variety of stakeholders including patient advocacy groups and not-for-profits, the pharmaceutical and medical device industry, healthcare professionals, academia and government.
- Utilise the expertise of stakeholders through working groups to identify and prioritise key issues, and to evaluate and propose initiatives that support the patient voice through enhanced involvement in the HTA process.
- Provide tools and resources for consumers, patients, industry and government that support education and the development of new methods and approaches to delivering patient input and patient-based evidence to be used in the regulatory and HTA processes.
Message from our Chair

When I attended the first Patient Voice Initiative (PVI) event – as a patient participant, the focus was on providing a forum for stakeholders to identify the current role of consumers in healthcare decision-making frameworks, and determine whether the patient view was adequately represented.

The response from all stakeholders at the event was resounding agreement of the need for enhanced patient involvement to ensure the Australian healthcare system meets the needs, values, and expectations of those who most depend on it. This quickly shifted the focus and scope of work for the organising committee. As a patient waiting for guaranteed access to a new therapy, I knew just how important and needed this work was.

Globally there is strong acknowledgement of the need and value of involving patients in healthcare decision-making. However, the challenge remains: How to best facilitate involvement that captures and values patient expertise in a robust, dynamic, and systematic way, while still offering timely access to treatments and therapies? The PVI steering committee hope this report will go some way in offering solutions to this challenge.

As the PVI team travelled to forums across Australia in 2016, speaking with patients and patient advocacy groups, it was evident that patients were interested in how decisions were made. Increasing interconnectivity, greater access to information, and shifting approaches to the delivery of healthcare and treatment pathways have all contributed to a growing expectation from patients that their skills, knowledge, and experiences will be formally heard and valued. They are no longer happy being passive recipients of healthcare—they want to know their needs and values are being considered as part of the processes critical to their health and wellbeing, and that they have opportunities for involvement. They also recognise that having input isn’t simply about making noise. Patients want their voices to be informed, impactful, and valued.
This echoes trends observed by those involved in healthcare decision-making. In Australia, consumer submissions to the PBAC almost doubled from 2013 to 2016. The Australian Government, and particularly the consumer advocate and deputy chair of the PBAC, Jo Watson, has acknowledged consumers’ increasing expectations for involvement. Accordingly, the government has offered consumers more opportunities for engagement and representation, with the introduction of consumer hearings to the PBAC process, an additional consumer advocate added to the PBAC, and the formation of the Health Technology Assessment Consumer Consultative Committee.

Clearly, patients are not alone in recognising the importance of their voices. As the financial burden for governments providing access to treatment increases with new treatment paradigms, now more than ever the consumer’s role in determining value—what treatments matter to them and why—is critical in ensuring our system aligns with patient needs and values.

One of the greatest revelations to me while working with PVI has been the support for patient involvement from all stakeholders. There is collective recognition that we must have a system that develops, brings to market, and provides access to treatments that align with the needs and values of patients and the broader community. I believe one of PVI’s greatest strengths to date has been bringing stakeholders together and forming a collaborative approach to identify the need for change and to offer the solutions contained in this report.

Some of this report’s recommendations are relatively simple and easily implemented solutions that simply require understanding of patient needs. While we acknowledge that other recommendations will take more time and require legislative change, we know that patients need these outcomes. The Australian healthcare system must not fall behind in its ability to deliver care and outcomes that matter to patients.

The generation of medications currently coming to market are breakthrough therapies that patients (like myself) have been waiting for our entire lives. The only thing more heartbreaking than not being able to treat disease is knowing there is a treatment that works in ways that matter to patients, but not being able to access it because those who don’t live with a disease have become the gatekeepers of what defines value. Innovative policy that gives patients a voice in determining what matters to them in the pursuit of treatment is equally as important as innovative science in patient’s ability to access treatment.

The Patient Voice Initiative will work with stakeholders to ensure this critical progress becomes a reality. We hope the recommendations contained in this report can inspire timely action that ensures Australia has a healthcare system that can learn from all stakeholders, but particularly those most affected by healthcare decision-making—patients. We want to ensure that patients have access to treatments that not only keep them alive, but allow them to live.
Why we want a greater patient voice in HTA?

Patients (or health consumers) have experience-based expertise gained from living with health conditions and using health technologies. Their unique knowledge is currently underutilised when decisions are being made about if, how and when we should use health technologies in Australia. Providing patients with the mechanisms to have a greater voice in HTA increases the relevance of HTA and reduces uncertainties by taking account of the real-life context of patients. Simultaneously, it has the potential to contribute to health literacy by improving patient understanding of HTA.

Having a patient voice in HTA means patients and carers understanding HTA, able to contribute knowledge that adds value to HTA, and feeling respected and heard.

While patients are sometimes included for democratic reasons, the Patient Voice Initiative believes that the goal of engagement should be much more than recognising patients' right to be involved and improving transparency.

Greater engagement will increase understanding of HTA and, in turn, increase the capacity of patients to make valuable contributions. Furthermore, it may strengthen the evidence-base for HTA, making the process more robust as it takes account of social values, ethics, and patients' and carers' needs, preferences and lived experiences. Finally, greater engagement before, during and after any HTA process may lead to enhanced decision quality at all stages.

Consumer Engagement...consumers and carers actively partner with health organisations in their own healthcare and in health policy, planning, service delivery and evaluation at all levels of the health system.\(^2\)

To achieve these goals, HTA in Australia needs to better include the patient voice by systematically using:

- **Patient participation** (two-way communication with patients, including patient input such as taking submissions and enabling patients to take part in HTAs, to enable committees and patients to learn from each other and solve problems before, during and after an HTA)

- **Research into patient aspects** (patient-based evidence, i.e. primary or secondary research into patients' experiences, preferences and perspectives using robust scientific methodology).\(^4\)

Over five years CADTH received 297 patients input submissions for 142 HTAs which were used in its Common Drug Review's scoping, assessment and appraisal phase. A study of 30 HTAs found these submissions provided 119 patient insights about health status, progress of recovering and health sustainability. They also helped frame HTAs and interpret other HTA evidence.\(^3\)
Summary of needs & recommendations

In 2016, the Patient Voice Initiative held one-day meetings in Sydney, Brisbane, and Melbourne to discuss how the patient voice could be improved in HTA in Australia. Patients, patient group representatives and other interested parties such as industry, researchers and government took part. Attendance figures were: Sydney (117), Brisbane (20), Melbourne (17).

The outcomes of those meetings were then presented at a meeting in Canberra, enabling participants to note a high degree of consensus about unmet needs and identify priority areas for improving the patient voice in Australian HTA.

Stakeholders’ needs which were not being met fell into four areas:

01 **Legislation and procedures**
Assurance that the participation of patients and patient groups in HTA will be supported and their input and patient-based evidence considered

02 **Communication**
Create a well-resourced unit to support and develop patient involvement and provide clear contact points and opportunities for dialogue

03 **Training and guidance**
Opportunities to learn in order to improve patient and patient group capacity to make valuable contributions to HTA

04 **Evidence generation**
Strategic tools and projects to build a robust evidence base about patient aspects, eg registries of patient experience
Stakeholders identified the following priorities:

1. Earlier engagement of patients in the medicines’ reimbursement process so that they are an integral part of the entire journey from the time a reimbursement application is first identified, rather than at the end of the process when the committee is imminently making a recommendation.

2. A dedicated consumer portal website that explains in plain language each committee’s purpose, processes and involvement opportunities, and provides comprehensive links to tools and advocacy groups.

3. An agreed, standardised and systematic approach to capturing and interpreting evidence that is relevant to patients in the decision-making process for the funding of medicines and other health technologies.

4. Activities to improve patient groups’ understanding of the HTA processes, decision-making and outcomes from committee meetings, and proactively provide feedback to patients on the value of the evidence contributed.
Participants in the workshops wanted to know that if they participated and provided evidence about patient aspects, that it would be considered. Furthermore, they wanted to know how it would be taken account of, what input and evidence would be welcomed, and how they would be supported and encouraged to provide it.

We recommend a change in the legislation to ensure HTA explicitly takes account of social values in addition to clinical and cost effectiveness, and includes consumer engagement throughout its processes. We recommend that HTA committees commit to taking account of patient input and patient-based evidence in their documented procedures and terms of reference.

Patient participation in HTA has been legislated for in Germany’s G-BA and IQWiQ since 2004, in Italy’s AGENAS since 2014 and in Taiwan’s NHIA since 2016.

Rachel Lamb
Type 1 Diabetic

“I believe [the patient voice] is one of the most crucial elements in healthcare decision making. The clinical data shows one part of a very large picture. My hbA1c may show the average of my blood sugars over a few months, but it doesn’t reflect the daily struggles, the mental burden or the psychosocial impacts of living with type one diabetes. The clinical data is extremely important, but my daily experience of living with my chronic condition is so much more than that.

Together with policy makers, clinicians, decisions makers, etc. you have the full picture. Without adequate patient input, effective choices that reflect the best interests of our society and health care system cannot be made.”
Patients and patient groups participating in the workshops sought:

- Clear contact points, e.g. contact details for consumer representatives and specific staff in a patient involvement unit
- A forum where patients and patient groups can discuss potential improvements to current systems
- A one-stop website portal where patient groups can access all information available to support patient involvement in HTA
- A registry of patient organisations to support communication so that committees can contact relevant groups to provide early alerts about submissions and patients and patient organisations can work together on submissions
- Earlier engagement and increased opportunities to interact
- An online calendar so patients and patient groups can see relevant topics and key dates and plan their work
- An alert system advising patient organisations when relevant submissions are posted to PBAC agenda or topics considered by other committees
- Dialogue with committees to update consumer comments form
- Formal plain language feedback on how patient input and patient-based evidence was taken account of and how decisions were made
- Formal feedback on patient group submissions about what was valuable and what information could have assisted the committee
- Improved communication about industry submissions and their content.

By consulting with patients when scoping HTAs, England’s NICE reduces the risk of using inappropriate comparators in economic models and increases its ability to ask the questions that matter to patients first time around.4

CADTH’s Common Drug Review enables patient groups to sign up for e-alerts and watch out for ‘Call for patient input’ emails.5
HTAi’s Patient and Citizen Involvement Interest Group publishes a wide range of educational resources which are designed to be adapted by local HTA bodies. It’s currently building a repository of materials from around the world which have been designed specially to help patients participate in HTA.6

Kathy Gardiner
Advanced Unresectable Metastatic Melanoma Patient. Kathy benefited from compassionate access to immunotherapy, Keytruda.

“As with most things in this grey area [of public reimbursement], the patient is left to invest long hours of research into becoming the expert of not only their own disease, but the world of drug access within Australia, often placing their own recovery in unpredictable circumstances to fight for the greater cause. Unless you are highly dedicated and have outstanding involvement and contribution within your designated Patient Advisory Group, you are left quite in the dark.

In my experience [with PBAC submissions], I have never received any formal feedback. Because of this lack of feedback, patients like myself hold on to the faith that “hopefully our voices were heard”, and the only way we know if they have been heard is if a successful approval is formally announced.

Patients want to be involved. Patients need to be involved, and patients are more informed now than they have ever been. Never has there been a more important time to have a more inclusive and consultative environment to ensure that the people of Australia are cared for, listened to and part of the conversation.”
03

Training and guidance

Opportunities to learn in order to improve patient and patient group capacity to make valuable contributions to HTA

Patients and patient groups in the workshop sought training and plain language guidance on:

- HTA processes, including key points for patient involvement, how different evidence is assessed and why participating is important
- Committee processes
- Preparing consumer comment submissions
- Preparing for consumer hearings
- Case studies demonstrating effective patient involvement in HTA
- Patient-based evidence including methods, links and how it will be considered by committee.

Patients and patient groups would also benefit from more information about sponsor submissions. Publishing the indication with the name of the medicine on the website and providing a consumer-friendly summary about the medicine would assist.

SMC’s website provides plain language guidance on the ways patient groups can collect and report patient information. Its site also offers education videos allowing people to learn from the experiences of other patient groups and see how committee meetings work. Open meetings enable patients and the public to better understand the process and their role. Additionally, consumer-friendly summaries about the medicines are made available to registered patient groups to help them better target their submissions.
SMC staff provide patient groups with feedback on their draft submissions to help them improve the quality. CADTH provides a letter giving feedback to submitting patient groups at the end of each HTA to enable them to know how their work was used and how future submissions can be improved.4

Lisa Briggs
Rare Cancer Patient

“The system is not currently designed to cater for the consumer voice adequately enough. It is far too confusing for an educated person and does not cater for the wider population enough. Cancer doesn’t discriminate, but unfortunately our ability to understand the complexity of lodging consumer comments or obtaining information regarding the process does.”

Vanessa Richards
Marfan Syndrome Patient, Rare Disease Advocate and Secretary of the Marfan Association of Queensland

“It would be beneficial for the community if there were a way for us as an association to help provide information to our community [about how to have input into the public reimbursement process], however for us to do that we would need to have access to the information… Personally as a rare disease patient, I feel lost in the system”
Evidence generation

Strategic tools and projects to build a robust evidence base about patient aspects, eg registries of patient experience

Participants wanted greater use made of research into patients’ needs, preferences and experiences. Using more patient-based evidence in HTAs can reduce the burden of information collection by patient groups and allow this information to be critically reviewed and assessed with evidence about clinical and cost effectiveness.

Participants wanted:

- Repositories and registries of patient experiences with treatments which could be used as data for HTA processes, eg a survey of patients’ experience of medicines which can be used in HTAs
- Guidance on what patient-based evidence will be considered and how it will be assessed and used.

HTAi’s Patient and Citizen Involvement Group has projects to develop rapid Qualitative Evidence Synthesis (QES) especially for rapid HTAs and is exploring the development of global repositories of patient experience for use by HTA bodies. These are projects Australian HTA bodies could work with. Additionally, in Scotland a researcher is working on a framework to allow health researchers not expert in QES to conduct QES.
Patient preference research methods (eg discrete choice experiments/conjoint analysis) measure the values of patients with a particular condition to explore how they perceive treatments and to understand what is most important to them. The methods can provide additional evidence when there are gaps in clinical trial or quality of life data, eg often the case when assessing treatments for people with rare conditions or small sub groups of patients. They can measure the value of non-health benefits, eg the importance of taking a medicine once a week instead of everyday or of an oral tablet being available instead of only an injection. And, they can provide evidence about the risk or side effects different groups of patients are willing to experience to gain a particular benefit in treatment. The results provide a patient value framework which can be used to benchmark all treatments (both new and existing).

Researchers are investigating how best to measure patient preferences for healthcare decision making. One of the largest research projects is the IMI PREFER research initiative in Europe which is considering how and when it is best to perform and include patient preferences in decision-making during the treatment development life cycle. PREFER will provide recommendations to inform guidelines for industry, regulatory authorities and HTA bodies.

www.imi-prefer.eu

Lisa Briggs
Rare Cancer Patient

“What it means to a patient to be able to access a drug can at times be life changing. For someone like myself with such a young family, it’s not just about the length of my life as much as it is also about the quality of my life. Being able to run around and play with my children, take them to the park or be a helper in the classroom are activities of daily life some might take for granted. But to be able to do that and smile at the end, is far more rewarding than you can imagine. These are the things we are grateful for... Patients have the rare ability to provide that unique perspective to the PBAC which no statistic can.”
Direct patient involvement has been critical to the success of PVI
Conclusion

Around Australia considerable expertise about living with conditions and using services and treatments resides within patients. However, it remains largely untapped throughout the life span of treatments. Meanwhile, HTA bodies are grappling with uncertainties that could benefit from this expertise while appearing inaccessible to many patients and patient groups who are often frustrated by a lack of transparency and decisions that appear inexplicable. As the Patient Voice Initiative listened at its workshops, it heard from patients and patient groups who wanted to actively engage with HTA bodies, but lacked key tools and resources to ensure their contribution added value. To better deliver healthcare that meets the needs and expectations of Australians, the Patient Voice Initiative recommends:

- Introducing legislation and procedures to ensure that patients and patient groups that take part in HTA will be supported and their input and patient-based evidence considered
- Improving communication with a well-resourced unit to support and develop patient involvement with clear contact points and opportunities for dialogue
- Developing further training and guidance so that patients and patient groups build their capacity to make valuable contributions, e.g. by ably addressing gaps and uncertainties in the evidence base
- Developing strategic tools and projects to build a robust evidence base about patient aspects which can be critically assessed and reduce the burden on patients and patient groups.

This report makes recommendations to improve the patient voice in HTA in Australia. They are based on the needs heard at Patient Voice Initiative workshops and will serve as guideposts for its future activities. However, this report is also an invitation to all those who want transparent and evidence-based HTAs throughout Australia that are informed by patient expertise to support the delivery of patient-centred healthcare. The Patient Voice Initiatives invites you to discuss this report with us and consider collaborating with us to achieve this goal at all levels across the health eco system.
Appendix
Steering Committee, Partners & Stakeholders

**PVI Steering Committee:**

Jessica Bean (Chair), patient advocate
Ann Single, patient involvement advocate
Kate Vines, Rare Cancers Australia
Simon Fifer, CaPPRe
Todd Stephenson, Janssen
Hayley Andersen, Bristol-Myers Squibb

**PVI recognises the valuable input of previous steering committee members:**

Laurie Axford, CaPPRe
Ian Olver, University of South Australia
Pete Murphy, Novartis
David Puller, Roche
Nicola Richards, MSD
Carlene Todd, Roche
Richard Vines, Rare Cancers Australia
Nathan Walters, Janssen
Zarli French, MSD
Ian Noble, Noble Consulting

**Industry Sponsors:**

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Amgen
Bayer
Bristol-Myers Squibb
CaPPRe
Janssen
MSD
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Pfizer
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Sanofi

**Report design:**

Max Kelsen

**Health Consumer Organisations:**

Cancer Voices
Centre for Community-Driven Research
Consumer Health Forum
Leukaemia Foundation
Lymphoma Australia
Macular Disease Foundation
Melanoma Patients Australia
Ovarian Cancer Australia
Rare Cancers Australia
Unicorn Foundation
References


Further Reading

HTA in Australia

HTA general information
