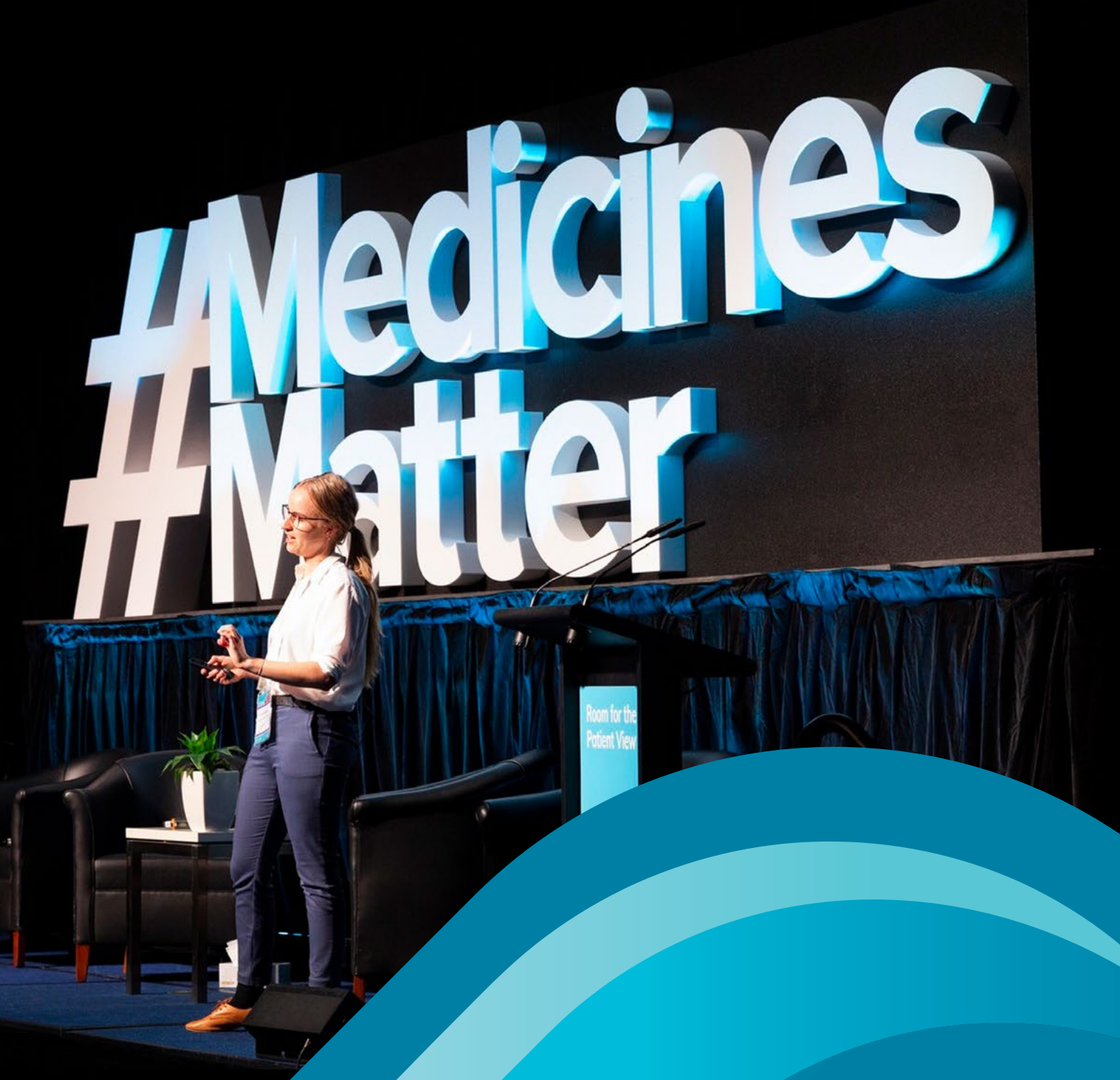
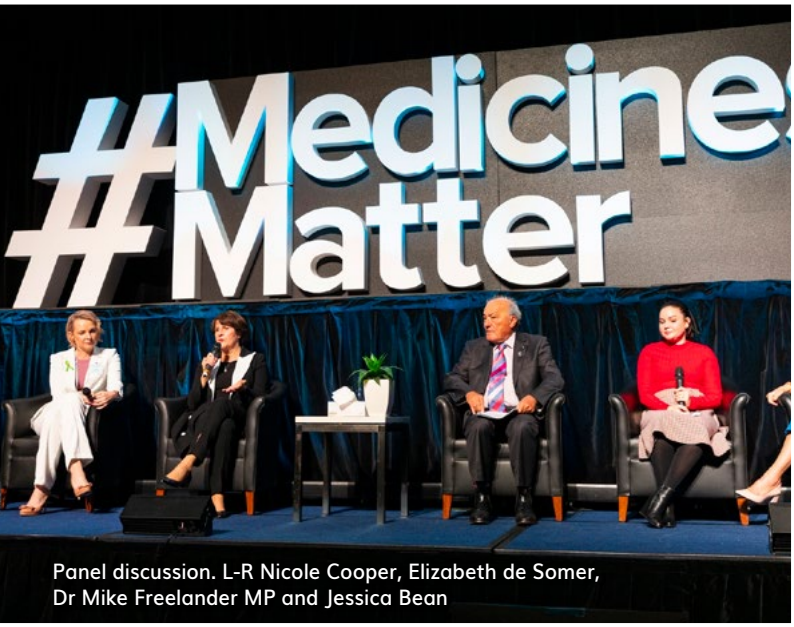


PharmAus

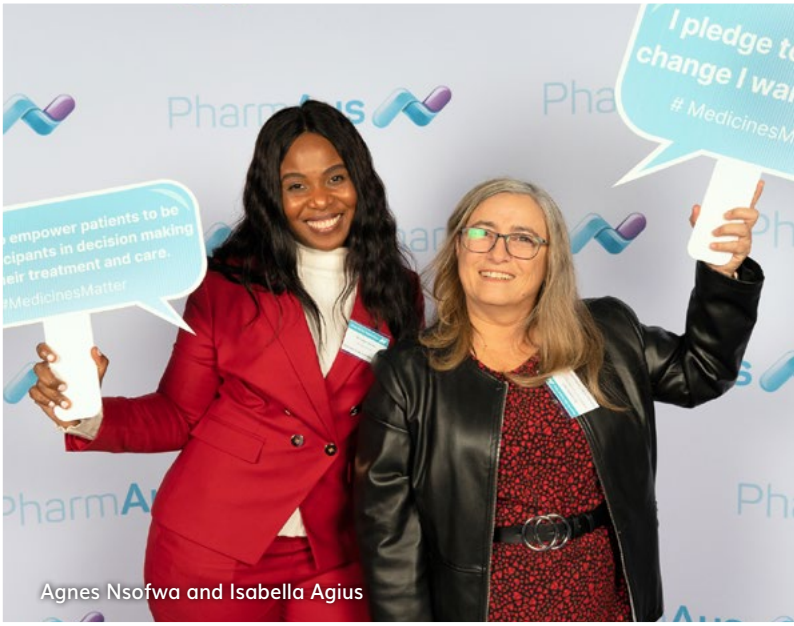
Presented by Medicines Australia

Room for the Patient View

Contents



Panel discussion. L-R Nicole Cooper, Elizabeth de Somer, Dr Mike Freelander MP and Jessica Bean



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evohealth

Room for the Patient View

PharmAus
Presented by Medicines Australia

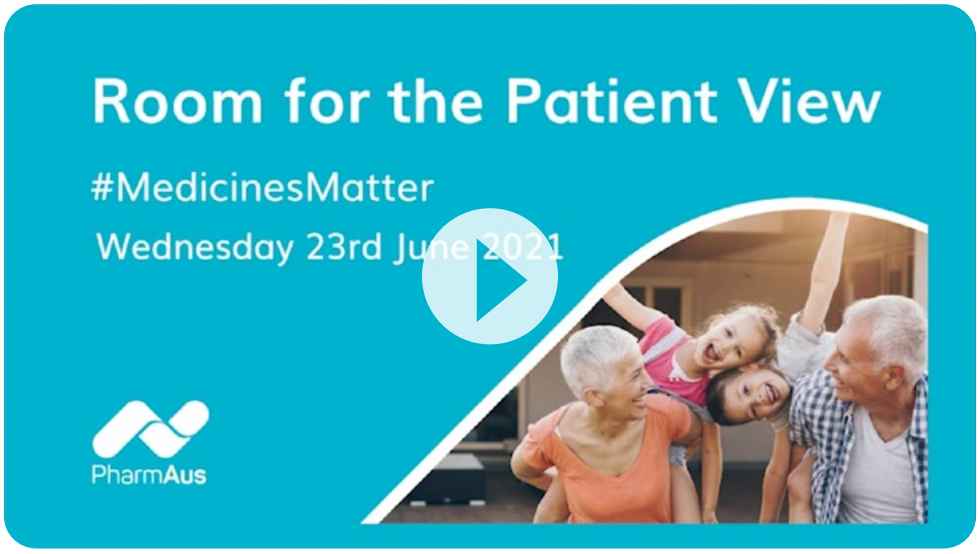
Room for the Patient View, a PharmAus event, was held on 23rd June 2021 in the Great Hall at Parliament House, Canberra.

At a time when the importance of health is firmly in the spotlight, the event brought together patient advocacy groups, Members of Parliament, Senators, parliamentary staff, media and representatives from across the health industry to explore issues that impact all Australians. The event saw 128 attend in-person and 126 watch virtually via livestream.

Master of Ceremonies, Tracey Spicer AM facilitated this important conversation. The evening featured two patient speakers, followed by a panel discussion with patients, clinicians, Dr Mike Freelander MP and Medicines Australia CEO Elizabeth de Somer. Also addressing attendees at *Room for the Patient View*, were the Minister for Health, the Hon Greg Hunt MP and Shadow Minister for Health, the Hon Mark Butler MP.

During the event, patients shared personal health experiences and the journey from diagnosis through to accessing treatment, including innovative medicines. Understanding what matters to patients in the health system was extensively explored, including how best to incorporate the patient view in the regulatory and reimbursement process, and how we can work to improve timely access to new and innovative treatments.

Room for the Patient ViewWrap-up video:



Four key themes emerged from the speakers and panel discussion, namely:

1. Embedding the patient voice from the beginning

The patient voice needs to be embedded throughout the health system and, when it comes to medicines, right from the start of the development lifecycle. This ensures what is relevant to patients can be captured and included in any future evaluations.

2. Supporting innovation in a rapidly changing landscape

We live in an age of constant and rapid changes in health technology, including innovative therapies. Our system needs updating and is not fit-for-purpose. Australia's system needs to adapt to support these changes and ensure patients have timely access to these life-changing treatments.

3. Minimise the time for patients to access new and novel technologies

Patients need earlier access to treatment. The rapid development and evaluation of COVID-19 vaccines around the world has shown what is possible when the global community works together.

4. Capturing the true value of health interventions

Australia's Health Technology Assessment (HTA) system does not capture the true value of what is important to patients and society. An updated system should reflect the important benefits that a healthy society brings to our economy such as increased workforce and social participation.

Each of these themes are explored in further detail in this report.

Speakers & Agenda

Welcome & Opening

Tracey Spicer AM

Master of Ceremonies

Patient Story

David Lockwood

NeuroEndocrine cancer patient

Kaitlyn Sapier

Multiple Sclerosis patient

Panel Discussion + Q&A

Jessica Bean

Cystic Fibrosis patient, Patient Voice Initiative President and Chair, consultant and coach

Nicole Cooper

Bowel Cancer patient and management consultant

Elizabeth de Somer

CEO Medicines Australia

Prof. Andrew Spencer

Head of both Myeloma Research Group Malignant Haematology and the Stem Cell Transplantation Service at The Alfred Hospital

Dr Mike Freelander MP

Paediatrician and Deputy Chair of the House of Representatives Committee Inquiry into approval processes for new drugs and novel medical technologies in Australia

Panellists took questions from the audience and from the virtual audience via an event app.

Closing Remarks

Hon Greg Hunt MP

Minister for Health and Aged Care

Hon Mark Butler MP

Shadow Minister for Health and Ageing

Discussion Themes

1. Embedding the patient voice from the very beginning

When it comes to medicine and what matters to patients, a recurring theme at *Room for the Patient View* was the importance of including the patient perspective throughout the entire process – from trial design until after the medicine is made available.

Speakers and panellists agreed that the patient voice should not be an afterthought or a 'box ticking' exercise. Multiple opportunities to better embed patient input in Australia's health technology assessment framework were discussed.

Include the patient perspective from the very beginning

Patient representatives spoke of the importance of developing patient relevant measures, including patient reported outcome measures (PROMs) and patient reported experience measures (PREMs) before clinical trials commence. This provides an important opportunity to focus trial design on outcomes and models of care that work best for patients.

"...we have to broaden the evidence base to ensure things that matter to patients are captured."

– Jessica Bean

The panel supported the collection of real-world evidence (RWE) throughout the trial and post-trial phases as a key mechanism to capture the reality of the patient experience. Elizabeth de Somer, Medicines Australia CEO, spoke about the need to incorporate the collection of RWE and relevant patient measures from the very beginning.

Professor Andrew Spencer, researcher and haematologist from the Alfred Hospital, echoed this, noting patient registries provide tangible opportunities to "tell us what is going on" but unfortunately, these critical datasets are not well resourced nor well accessed by decision makers.

Quality of life is more than just a numerical outcome

Embedding the patient voice as early as possible was also thought to facilitate downstream decision-making. Jessica Bean, a patient and medicine access advocate, suggested embedding the human experience much earlier and across the clinical trial lifecycle would mean the aspects that matter the most to patients are included prior to the Health Technology Assessment (HTA) process. The need to capture the patient voice in these processes was a strong focus for the group.

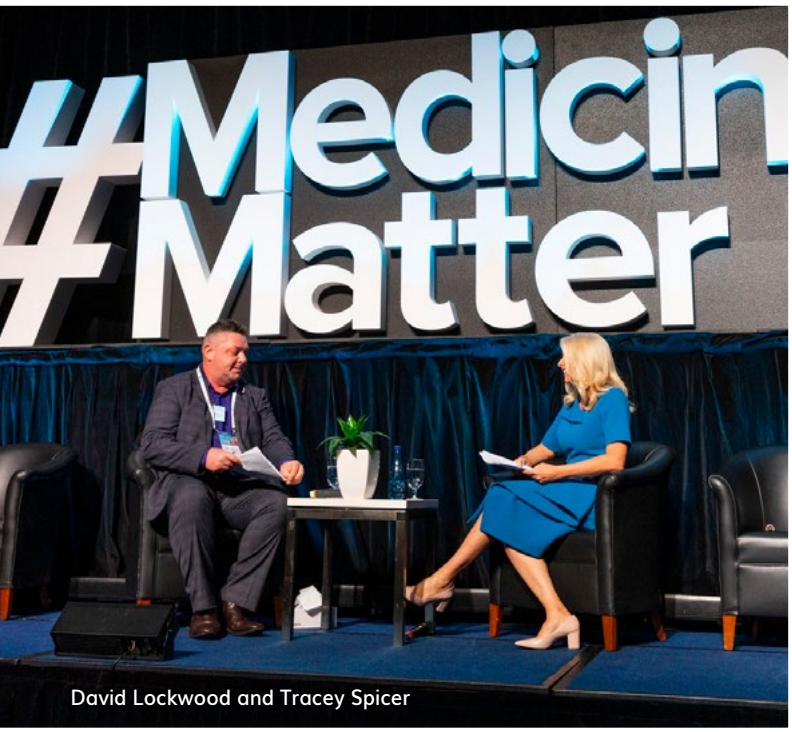
"we have to see [patient] stories... Our hope is not to look at numbers at end of clinical trial, our hope is to have more life, quality life."

– Jessica Bean

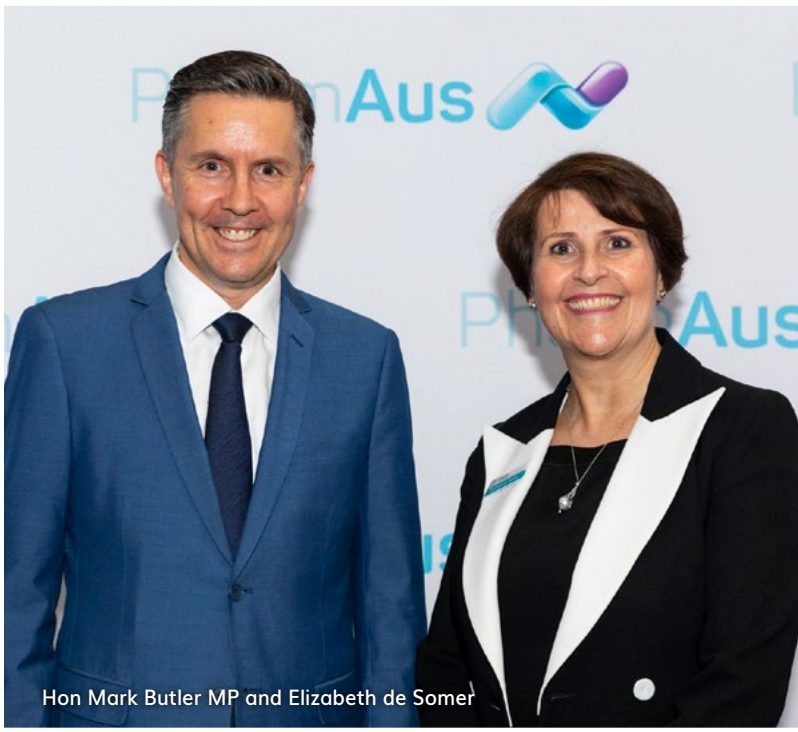
Patient advocate and bowel cancer survivor, Nicole Cooper, echoed the need for embedding the patient voice into these processes.

"Numeral measures [from clinical trials] ...do not have our personal experience attached to them. When considering a drug, [we need to] speak to patients with lived experience."

– Nicole Cooper



David Lockwood and Tracey Spicer



Hon Mark Butler MP and Elizabeth de Somer

Patient-centric models of care

Beyond regulatory and reimbursement processes, patient representatives and panel members all noted the need to embed the patient voice into diagnostic and treatment pathways to enable best practice care.

Patient representatives Kaitlyn Sapier and David Lockwood spoke of the difficulty in accessing consistent and timely care and the inherent complexity in navigating Australia's healthcare system. As a result, many patients struggle to get a timely diagnosis. This is a particular issue for patients with rare diseases and for those living in rural and remote areas with limited access to specialist care.

"I received an early diagnosis, but I had to push for it."

– Kaitlyn Sapier

Both patients and panel members highlighted there are already working models that "think outside of the box" and increase patient input. Dr Mike Freelander, MP and paediatrician, cited the McGrath Breast Care Nurses as an important case study for decision makers to consider, allowing time for patients to discuss issues and convey information from a patient perspective back to the health system.

Education enables critical patient input

Patient education was another important theme of the night. Patient representatives David Lockwood and Kaitlyn Sapier each detailed their long journeys to diagnosis, and how they continue to inform and educate themselves on their care and treatment. Current models being developed to enable patient input, such as the Patient Voice Initiative and the Continuity of Care Collaboration were highlighted.

When it comes to understanding Australia's regulatory and reimbursement system, the consensus view was a lack of understanding and education was a critical barrier to patient input. Jessica Bean highlighted the importance of patient education to facilitate patient involvement in PBAC considerations. Jessica considered that not understanding the HTA system, and how to provide information on "what isn't being captured", was a significant impediment to greater patient participation. Patients need to be better equipped to understand the process so they can inform decision makers about "what is missing... and what they want to see represented" in submissions.

2. Supporting innovation in a rapidly changing landscape

The new wave of innovative treatments and the changing landscape of treatment options was a consistent theme of the event. Panel members and patient representatives highlighted the shift in approach required to accommodate these upcoming innovative treatments, particularly those that have the potential to prevent further deterioration or even reverse the effects of previously devastating and incurable conditions.

Patient representatives spoke of innovative medicines that can now modulate genes and reduce lesions in multiple sclerosis patients and other medicines change people's lives. Jessica Bean shared broadening compassionate access to a novel modulator therapy for cystic fibrosis had, for the first time, led to patients like her being removed from the lung transplant list. For the first time, she has been able to travel on an aeroplane without the need for an oxygen canister.

"Because of [an] innovative medication, I can walk around up here and talk to you today."

– Kaitlyn Sapier

Elizabeth de Somer spoke about the role of industry in bringing these innovations to patients noting we must not lose sight of the fact these innovations require substantial high-risk upfront investments and that billions of dollars may be invested into research that fails. Maintaining a medicines industry that can thrive means further reinvestment into more innovation and subsequent downstream benefits for patients.

Mark Brooke, Elizabeth de Somer and Wes Cook

Tracey Spicer

Challenges bringing innovations to patients

Elizabeth de Somer and the Shadow Minister for Health and Ageing, Hon Mark Butler MP, both spoke of the challenges associated with the rapidly changing nature of therapeutics and the move towards personalised therapies within the context of our current healthcare system.

Dr Mike Freelander MP and paediatrician, highlighted how the delivery of some of these therapeutics, as either inpatient or outpatient, added complexities given the different processes and funding associated with Australia's federated health system. There was a view the system was outdated and not fit-for-purpose for innovations, with particular emphasis on the HTA process.

The panel concurred that when introduced in the 1990s, Australia's HTA system was gold standard, but times have changed. Especially when compared to other similar countries, it is evident that Australia's system and processes urgently need to be adapted to bring innovations to patients in a timely manner. The panel members expressed their hope the House of Representatives Inquiry into approval processes for new drugs and novel medical technologies in Australia would provide some recommendations and a way forward to address some of these challenges.

"We must do better because this is 21st century medicine."

– Dr Mike Freelander MP

These innovations also require innovative ways of working together, including within the pharmaceutical industry itself. Professor Andrew Spencer recognised in his field of cancer research, there was a particular need to better work together to ensure combination therapies can reach patients. Elizabeth de Somer noted "it's not just unwillingness to share, but inability in legislation to [allow] for information sharing." This sharing of perspectives from the panel helped support a broad discussion on consultation, input and transparency.

Review of the National Medicines Policy

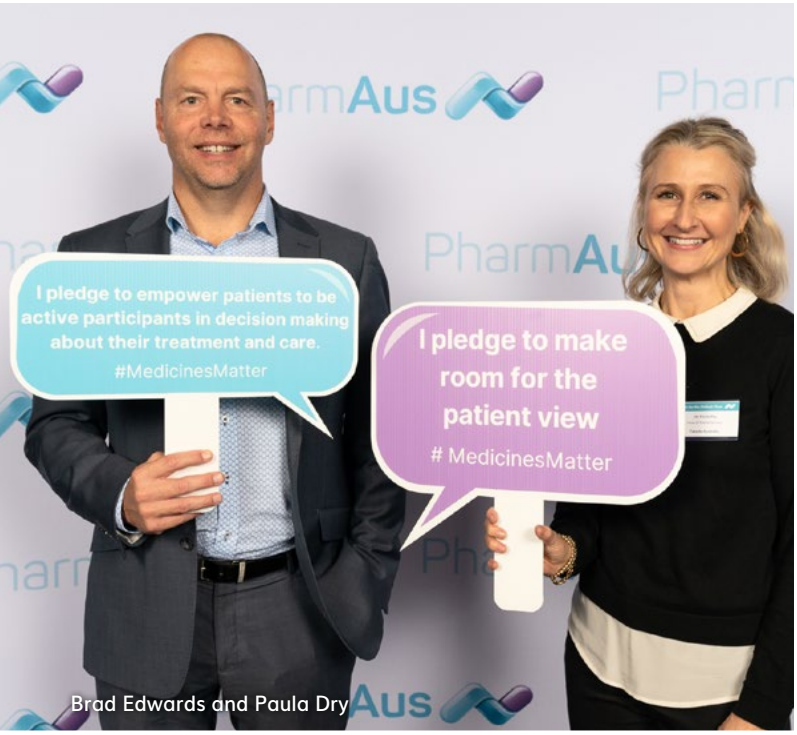
The Hon Mark Butler MP commended the objective of the National Medicines Policy (NMP) to "ensure affordable and timely access to medicines" as noble, but rapid changes in the industry towards personalised treatments and price reforms have put it under pressure.

The Hon Greg Hunt MP highlighted Government efforts to provide access to innovative medicines through the $2.8 billion New Medicines Funding Guarantee and emphasised the review of the NMP will provide opportunity to ensure future iterations are fit for 21st century therapeutics.

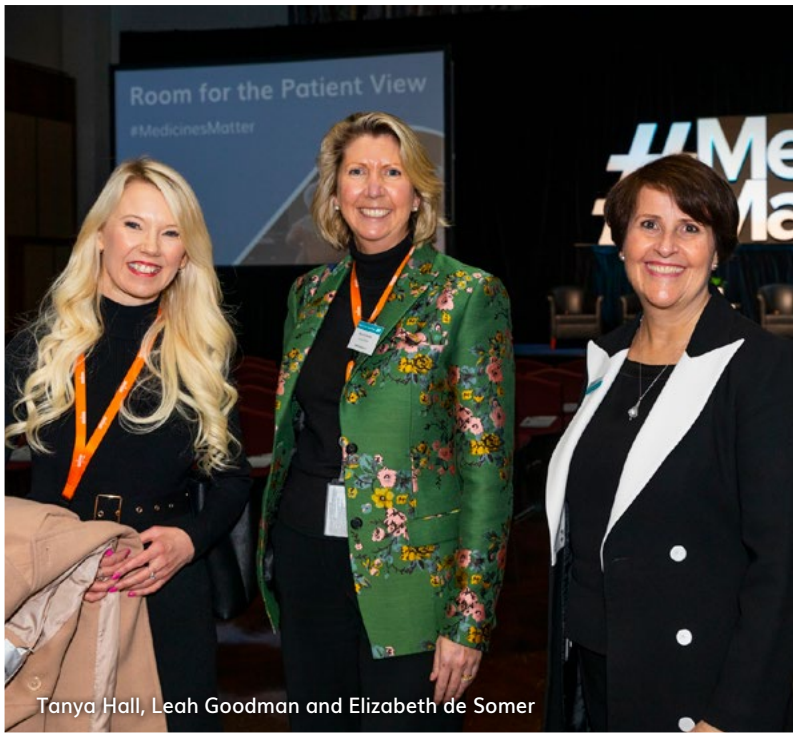
During his closing remarks, the Minister declared the review of the NMP, previously announced in October 2019, would be a nine-month process, starting on the 1st of August 2021. This was very pleasing to hear, as many stakeholders at the event have been calling for this for some time.

"The NMP has held us in good stead for 20 years. On the 1st of August we will start the review. We will do it in 9 months, consult with you and lay down an NMP for the next 20 years."

– Hon Greg Hunt MP



Brad Edwards and Paula Dry



Tanya Hall, Leah Goodman and Elizabeth de Somer

3. Minimise the time for patients to access new and novel therapies

In Medicines Australia's 2020 *Medicines Matter* report[1], on average, Australian patients have to wait over a year between medicines being deemed safe and effective and then being made available on the PBS. In fact, Australia ranks 10 out of 11 comparable OECD countries for the average time from registration to reimbursement. After receiving a positive PBAC recommendation, it takes another 210 days for a product to be listed on the PBS.

Speakers and panellists acknowledgedit is important to have a modern, robust HTA system to enable access to safe and effective treatment. However, the process in Australia is lengthy and duplicative. Individual country regulatory authorities appraise and consider medicines and vaccines with similar but distinct processes. Although Australia broadly looks to recommendations provided internationally, e.g., through the United Kingdom's National Institute for Health and Clinical Excellence (NICE) or the Food and Drug Administration (FDA) in the United States, there is still opportunity to enhance information sharing and cooperation across countries. The speakers noted, however, that during the pandemic, this collaboration has dramatically improved and , more broadly, there were tentative signs of better cooperation between approval agencies around the world (e.g. EUnetHTA guidelines).

The speakers concluded reforms to enhance information sharing and reduce duplication of effort should be encouraged to help bring innovations to patients sooner.

Nicole Cooper, Zarli French and Jessica Bean

Opportunities for early access

Throughout the discussion, all panel members reflected on the benefits associated with earlier access to care. Early access gives hope to patients and provides options, including valuable time in which other treatments might become available. Consistent with findings presented in the *Medicines Matter* report, the Minister acknowledged although the time from registration to reimbursement had improved in recent years, there was still work to be done.

1. Medicines Australia, 2020. Medicines Matter: Australia's Access to Medicines 2014-2019 http://www.medicinesaustralia.com.au/wp-content/uploads/2020/11/Medicines-Matter-Access-Report.pdf

Elizabeth de Somer believes the gold standard for Australia should be that there is no time difference between registration to reimbursement. She noted Medicines Australia is working closely with the Government towards this goal.

Clinical trials provide options and potential early patient access to innovative treatments. Although it was thought Australia is an attractive place for trials, particularly now when COVID-19 numbers are low relative to other settings, Professor Andrew Spencer argued most trials are very US-centric and focused on collecting data for FDA approval. Given this focus, he emphasised in his discipline, there are adverse domino effects associated with delayed access to novel therapies.

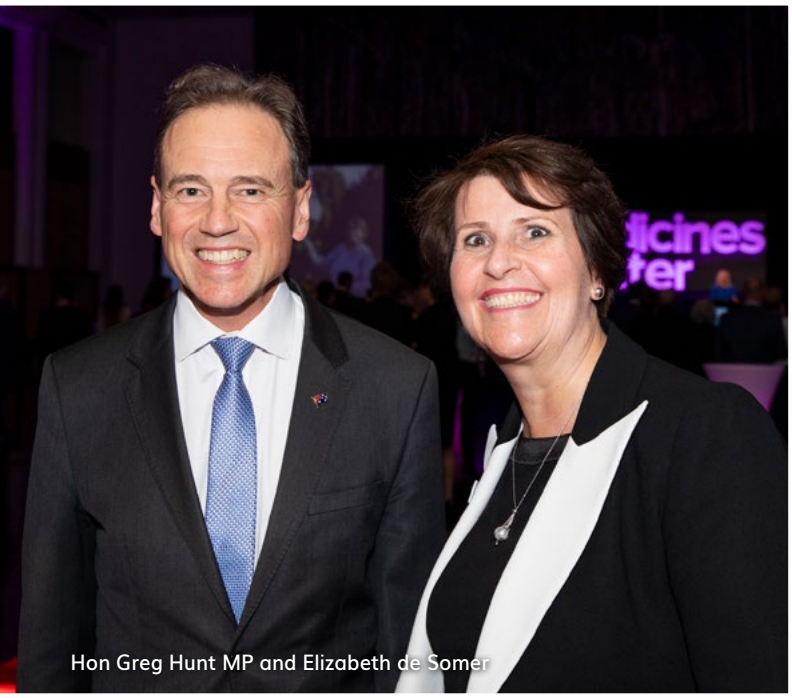
Trials are now being conducted in the US with comparator arms using treatments that are not available in Australia. It was noted regulatory arrangements for clinical trials are overly complex and inconsistent across states and territories. Altogether this could mean it is even harder to get these trials in Australia and even more difficult to provide patients with early access to innovative treatments.

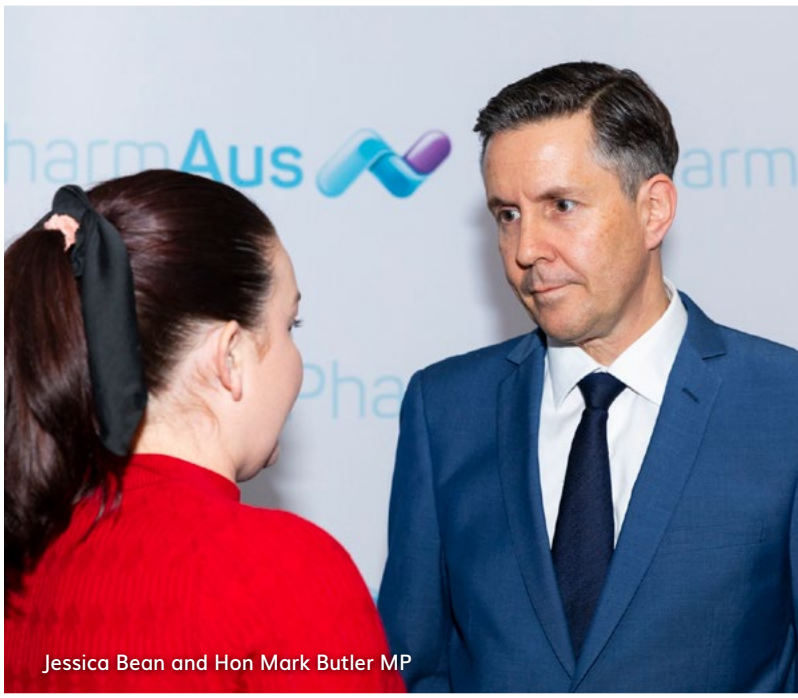
COVID-19 vaccines as a case study

Speakers identified in response to COVID-19, the pharmaceutical industry had "really put competition aside" to work together. It was concluded further industry collaboration, as well as legislative change to promote this collegiate behaviour, should be encouraged.

The panel acknowledged the processes in expediting approval and access to the COVID-19 vaccine were exceptional and in order to see similar timelines for other treatments, appropriate government resources are required. The rapid development of COVID-19 vaccines highlighted the pharmaceutical industry can step up to the urgent challenge.

These challenging times during the pandemic were seen as a rare event where identifying solutions, including a vaccine, became relatable and a problem for the global population.


Hon Greg Hunt MP and Elizabeth de Somer


Jessica Bean and Hon Mark Butler MP

4. Capturing the true value of treatment

There was a recurring theme at Room for the Patient View that regulatory processes need to capture the true value of treatment, including medicines and vaccines, in this rapidly changing landscape. The panel and patient advocates highlighted the rigidity of the current HTA processes in particular, noting some of the HTA constraints, such as limiting considerations to only "relevant healthcare costs", is not representative of the true value some therapies can bring to patients.

Value beyond cost-effectiveness

There was extensive discussion around the misalignment between the positive societal impacts some treatments can deliver and what is measured and deliberated upon in traditional HTA processes.

Some of the key ancillary costs are currently not systematically considered in Australia's approval processes discussed included:

- The broader impacts on families and society, from patients to families and carers. Current HTA processes cannot capture the value associated with patients, partners or carers being able to go back to work, school, or otherwise participate in society.

"Access to the best available healthcare not only benefits us as individuals, but also strengthens our community as a whole."

– Dr Mike Freelander

- Patient experience with receiving and undergoing different types of care e.g., the time to have different procedures or get to places where treatment is delivered, method of delivery, such as tablets vs in-hospital intravenous procedures.
- Social services costs and broader costs beyond immediate healthcare costs, including other government costs, such as disability support and carer payments.

"...there is value in medicines that goes beyond cost effectiveness [that] we don't take into account... [the] broader impact on family and the economy."

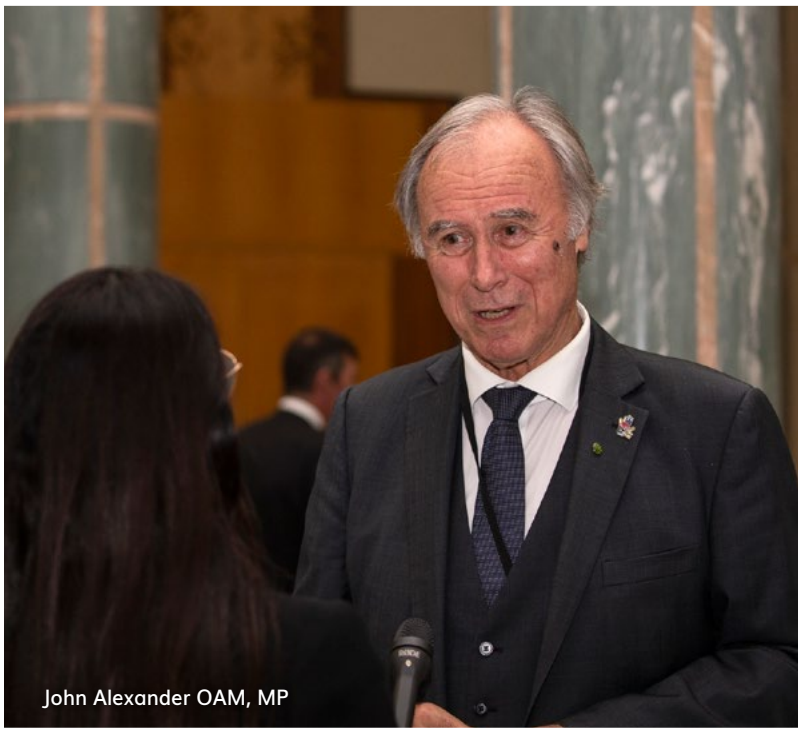
– Elizabeth de Somer

Jessica Bean spoke about her own experience when accessing an innovative drug that completely changed her life: "...early last year I was talking with the lung transplant team, and since being able to access a new modulator therapy, today is the first time I can travel without bringing an oxygen concentrator or nebuliser with me. This stuff you can't capture in data."

Nicole Cooper echoed these sentiments: “An extra week, an extra month or an extra year of life is valuable.”

The underlying challenges around to whom different treatments are targeted was also discussed, with speakers suggesting individuals with rare diseases or severe unmet need should be particularly considered, given a historical lack of treatment options.


Dr Mike Freelander MP


John Alexander OAM, MP

The need for change

Panel members concluded there was a need to systematically capture the patient voice to better understand broader societal impacts and the true value of therapeutics. More broadly, the panel members emphasised the need for a “complete paradigm shift” and real structural and cultural reform. Elizabeth de Somer captured this sentiment noting, from a Medicines Australia point of view:

“Our biggest message is about health being an investment, rather than a cost. Our health system is seen as a cost and something to be managed and contained, not seen as an investment in our future.”

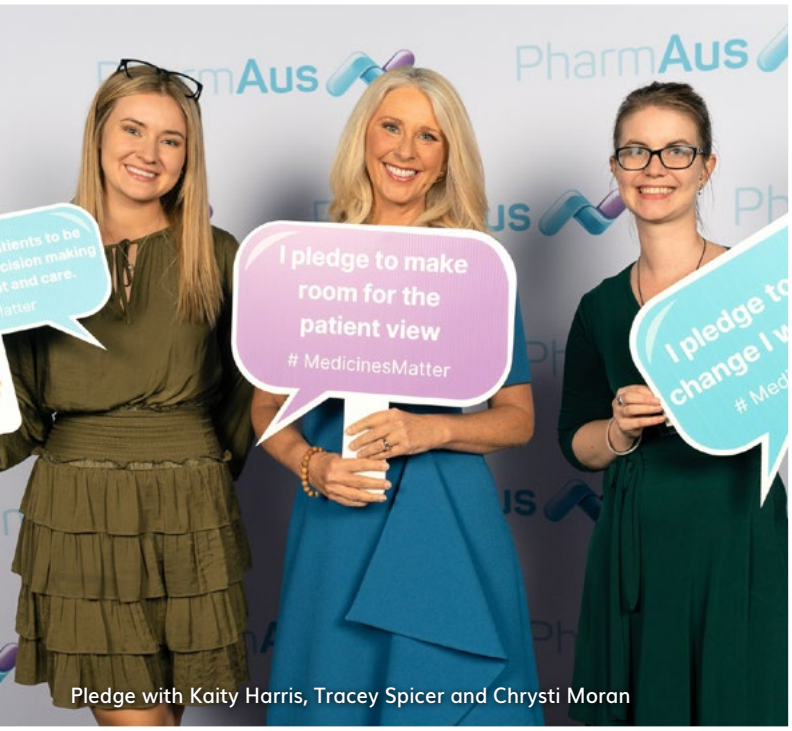
– Elizabeth de Somer

The panellists also hoped the current House of Representatives *Inquiry into approval processes for new drugs and novel medical technologies in Australia* would present recommendations to adapt regulatory processes to better align with societal expectations and capture the true value of therapeutics.

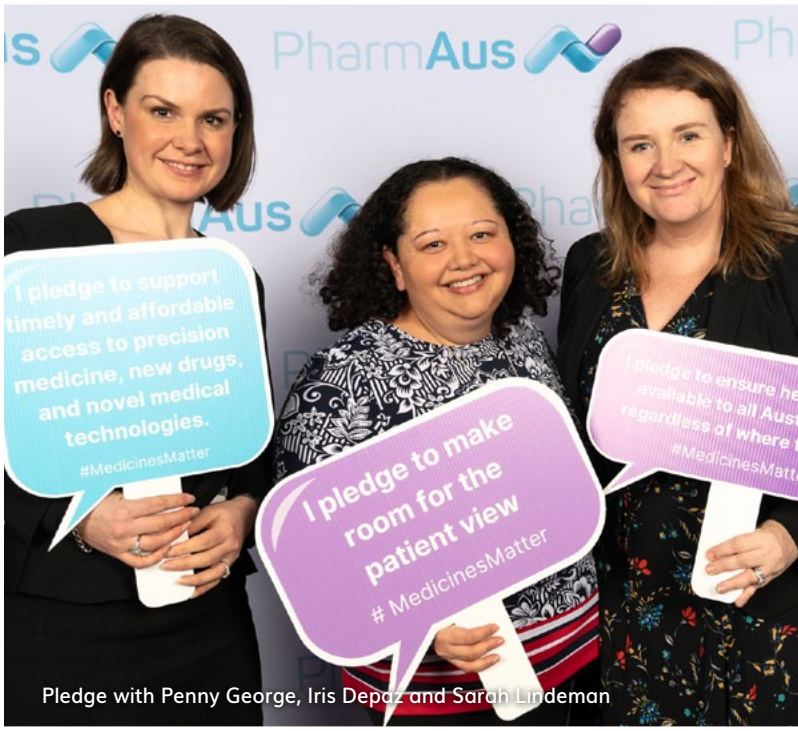
After the panel discussion was concluded, all 128 attendees (59 of whom were patients or patient group representatives) mingled and networked in the Great Hall.

Attendees were asked to select a favourite pledge via the event app or by taking a picture:

- I pledge to be the change I want to see
- I pledge to make room for the patient view
- I pledge to empower patients be active participants in decision making about their treatment and care
- I pledge to ensure healthcare is available for all Australians, regardless of where they may live
- I pledge to support timely and affordable access to precision medicine, new drugs and novel medical technologies



Pledge with Kaity Harris, Tracey Spicer and Chrysti Moran



Pledge with Penny George, Iris Depaz and Sarah Lindeman

Overall, "I pledge to empower patients be active participants in decision making about their treatment and care" received the most votes.

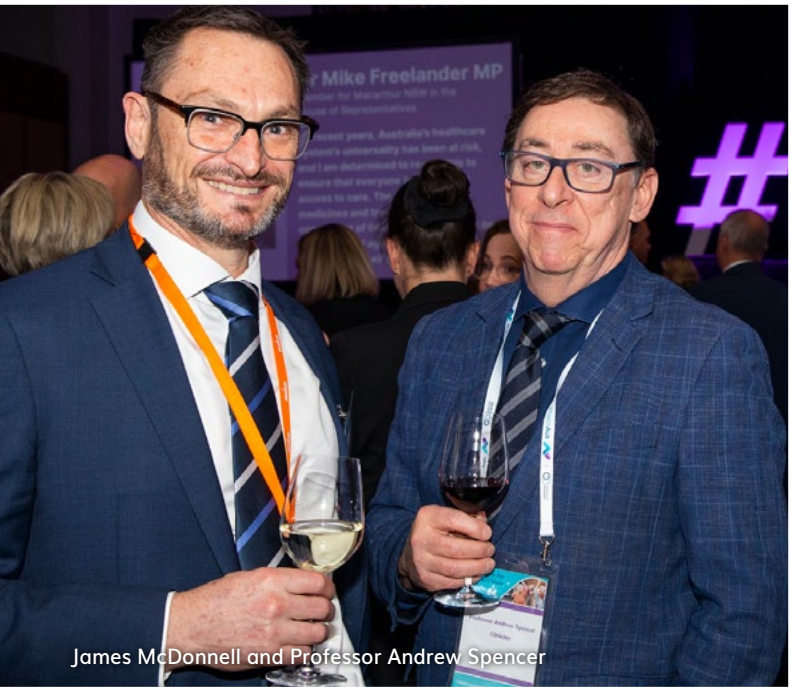
Attendees who attended in-person and virtually, provided overwhelmingly positive feedback about the event overall. Many expressed their gratitude towards the patient speakers sharing their stories and said the "key points about patient participation were valuable".

Suggestions for improvement for the next event largely revolved around enabling a deeper discussion on solutions and articulating actionable steps on "what exactly stakeholders can do working together to solve problems". "The whole concept was excellent" said one comment. "It was wonderful to have the opportunity to network and share information".

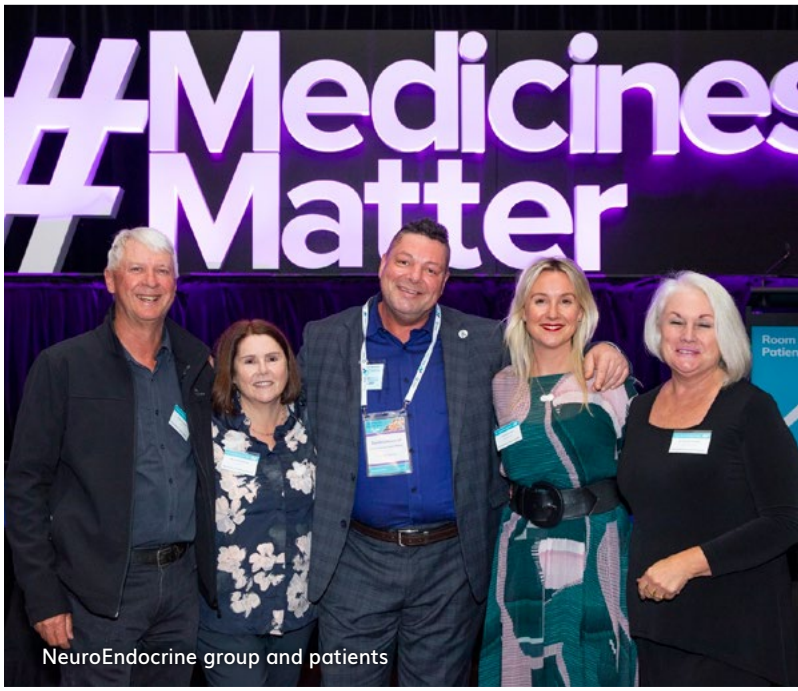
Next Steps

Room for the Patient View highlighted a range of opportunities for enhancing patient input to Australia's HTA process.

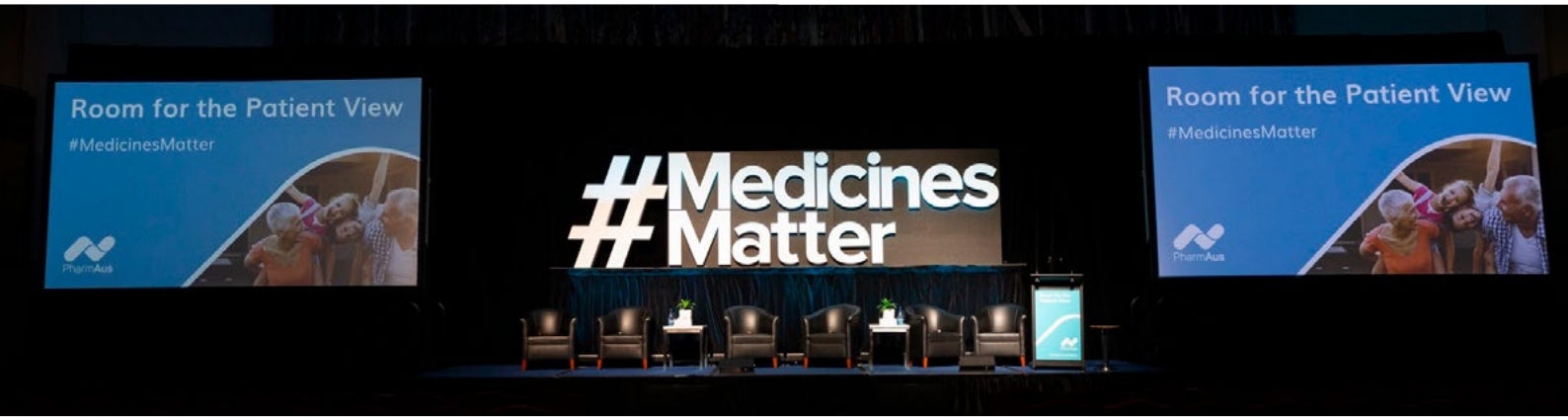
Medicines Australia is eager for the momentum of these conversations to continue. We look forward to the recommendations of the report from the House of Representatives *Inquiry into approval processes for new drugs and novel medical technologies in Australia* as well as participation from industry, patients and their representatives in the forthcoming review of the National Medicines Policy. Australian patients deserve no less.



James McDonnell and Professor Andrew Spencer



NeuroEndocrine group and patients



About Medicines Australia

Medicines Australia represents the discovery-driven pharmaceutical industry in Australia. Our members discover, develop and manufacture prescription medicine products, biotherapeutic products and vaccines that bring health, social and economic benefits to Australia.

Our vision is to make a real difference to the health of Australians by ensuring they have access to world-class medicines when they need them.

Medicines Australia represents the innovative medicines industry by:

- Engaging with Members of Parliament and government departments, the Australian Medicines Industry, consumer groups and health professionals to develop health and industry policy
- Building and maintain relationships with government for fair reimbursement of medicines (through the PBS) to ensure the continuation of a viable medicines industry
- Administering the internationally recognised Medicines Australia Code of Conduct which sets the standard for the ethical marketing and promotion of prescription medicines
- Working with other health professional and health consumers on issues of mutual concern
- Providing specialist advice to member companies
- Educating the community about industry activities.

For more information about Medicines Australia visit www.medicinesaustralia.com.au

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