AUSTRALIAN PATIENT ADVOCACY ALLIANCE

Submission to the Standing Committee on Health, Aged Care and Sport inquiry into the HTA Process in Australia

EXECUTIVE SUMMARY

The Australian Patient Advocacy Alliance (APAA) welcomes the Parliamentary Inquiry into approval processes for new drugs and novel medical technologies in Australia and is grateful for the opportunity to contribute and shape the Inquiry's deliberations.

The APAA is an alliance of Australian consumer health groups – collaboratively representing the voices of over 15 million Australians living with chronic conditions – dedicated to pursuing policy change. Together, we speak with one voice to improve access, break down barriers and drive innovation that improves health outcomes for all Australians.

Our shared ambition is to improve the health and wellbeing of all Australians through a health system that puts the consumer at its heart; one that draws on the varied experience of Australians living with health conditions, and embeds consumers at every step of the policy making process.

We thank the Minister for Health, the Hon. Greg Hunt MP, alongside the Standing Committee on Health, Aged Care and Sport for their leadership and foresight to critically assess our current health technology assessment (HTA) process. We acknowledge that our regulatory process (TGA and PBAC) is robust and trustworthy but we are highly supportive of this initiative to identify and support opportunities for reform to ensure the system is fit for purpose, embracing continual improvement and equitable and timely delivery of innovative medicines to consumers.

Consumers and their representatives play a critical role in pursuing health reform to better health outcomes by connecting the user of the health system with those who shape it. As such, consumers see the HTA process from a unique angle and our wealth of knowledge and real-life experiences must be incorporated in the process to ensure outcomes benefit those in need and address the concerns of those directly affected. It is vital that the patient voice is part of the Parliamentary Inquiry and subsequent review if the Terms of Reference are to be adequately addressed and health outcomes for all Australians are to improve.

To improve Australia's HTA process and bring it in line with more advanced international models, particularly regarding consumer engagement, innovative research and timely access to new medicines, we implore the Inquiry to explore the following recommendations:

1. **A health technology process that puts patients at its core**

Reform of the system to earnestly embed the voice of the patient by prioritising consumer co-design and consultation as a core fundamental in each step of the HTA process.

This reform should address inequities for those from vulnerable or disadvantaged populations including CALD groups, regional and remote Australians and First Nations Peoples. It is recognised that these are people who are traditionally left out of the loop in clinical trials and other related activities yet are often at highest risk of chronic conditions.

2. **Optimise the funding envelope with streamlined governance and a focus on outcomes**

Make the most of the funding envelope by implementing improved centralised governance through the establishment of national centralised bodies for disease control and prevention, ethics, and clinical trials that focus on oversight of outcomes.

3. **Improve HTA process and timeliness to bring treatments more rapidly within reach of all Australian patients**

Hold conversations on the public funding of a medicine when the drug is 'in market' to improve speed of access and to enable collation of representative data to demonstrate therapy outcomes more accurately.

4. **Further strengthen the transparency of the process that makes new therapies available**

Provide those directly affected – patients and clinicians – with appropriate, clear, accessible publicly available information on HTA processes plus updates and feedback throughout the process.

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OUR VISION

1. **A health technology process that puts patients at its core**

We seek reform of the system to strengthen and earnestly embed the voice of the patient as a core fundamental throughout the HTA process. Genuine stakeholder engagement and co-design will enable a system that is fit for purpose, meets consumer need and is designed for longevity. Establish a mechanism to strengthen consumer co-design and consultation and sharing of real-life evidence at every step before and during the process.

Current issues:

- There is a **lack of inclusion of consumers and consumer organisations at all steps in the HTA process**.
  - Currently there is no formal mechanism for pharmaceutical companies to inform consumers/consumer organisations regarding submissions.
  - Additionally, patient organisations are not always involved in research design and choices regarding clinical trials are currently clinician not consumer driven. Consequently funding often does not go to what consumers really want and need.
- There are **few patient specific measures included in evaluation**. We note that Patient Reported Outcome Measures (PROMs); Patient Reported Experience Measures (PREMs); Quality of Life scores; GAD7 or PHQ9 are not collected during research and clinical trials or included in the HTA process.

Proposed solutions:

a. Implement a **consultative mechanism to co-design process improvements** that will lead to increase stakeholder engagement. This may include encouraging pharmaceutical companies to inform and include consumers in the sponsored TGA and PBAC submissions. However, it is noted that inclusion of this in the process would require guidelines to safeguard consumer/consumer organisation independence.

b. Work collaboratively with consumers and consumer organisations to **improve consumer engagement in clinical trials**. This may include: reimbursing patient organisations for their time and involvement in the process; consumer inclusion in trial design including consideration for location barriers; and establishment of a register for the community to request or access trials to highlight research need.

c. Ensure the **inclusion of patient measures** and real-live evidence in the HTA process including clinical trials.

WE NEED: A consultation mechanism that genuinely places value on the consumer voice in creating an HTA process, including clinical trials, embracing consumer co-design with patients and patient organisations collaborating with Government Departments and Pharmaceutical companies. Additionally, we seek recognition of the importance of patient measures in trials and evaluation.

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2. **Optimise the funding envelope with streamlined governance and a focus on outcomes**

The APAA want to see the government make the most of the funding envelope by implementing improvements to the clinical trials system and enhanced centralised governance through the establishment of national centralised bodies for rare diseases, ethics, and clinical trials that focus on oversight of outcomes.

Current issues:

- **Support for improvements in clinical trials are patchy:**
  - **New clinical trial techniques are not valued** in the reimbursement process such that innovative approaches to clinical trials (N of 1, adaptive, organoids, basket trials) are not encouraged and incentivised; double blind placebo clinical trials are still seen as the only option or 'best practice'.
  - **Clinical trial volume is a disincentive** as Australia is often not seen as a country of choice due to population number and

there is a lack of inducement or expectation for researchers to link patients to global trials.
  - There is a deficit of government support for many **disease specific clinical trial networks**. These networks often support Investigator-led trials that address patient priorities and have strong consumer partnerships (as opposed to Industry led trials that more commonly support industry priorities).
  - There is **no national infrastructure** for clinical trials and no streamlined national ethics approval process
- **The HTA process is limited regarding mechanisms to innovate**: treatments are not personalised and precision medicine is not embraced; repurposing of existing treatments are not incentivised; acceleration pathways don't exist and rare diseases that don't meet the 'orphan drug' definition have no special approval pathways.

Proposed solutions:

a. **Streamline and nationalise processes plus improve governance and oversight** by establishing a suite of national entities including a Central Ethics Committee for research and clinical trials, an Office for Rare Disease and a national clinical trials registry
b. Establish **pathways for submissions where there is no sponsor** but benefit and patient need can be demonstrated
c. Establish a government supported program to **embrace new research technologies** and encourage them as part of the drug approval process
d. Implement a range of **improvements to clinical trials** including:
   i. Incentivise the inclusion of Australia in international clinical by providing benefits such as an expedited HTA approval process when Australian data is utilised
   ii. Creating a template for consumer organisations to establish a data registry for pre and post marketing surveillance
   iii. Embrace innovative clinical trials that include rural and remote communities.
   iv. We seek specific additional funding for the Australian Clinical trials Alliance (ACTA) (https://clinicaltrialsalliance.org.au/) to fulfil some of the proposed solutions
   v. Encourage and incentivise Not for Profit (NFP) consumer organisations to aid clinical trial participant recruitment and to establish Clinical Trial Networks.
   vi. Place added value or a 'positive weighting' on clinical trials done in Australia
   vii. Review outcomes of trials as part of funding process
   viii. Fund research into 'evidence gaps' for rare diseases

WE NEED: Efficient use of funding by implementing a range of improvements to the clinical trials system and enhanced centralised governance through the establishment of national centralised bodies for rare diseases, ethics, and clinical trials that focus on oversight of outcomes.

3. Improve HTA process and timeliness to bring treatments more rapidly within reach of all Australian patients

Australians must be able to access the therapeutics they need no matter how much they earn or where they live. However, technology is outpacing our system's capacity to evaluate and fund innovative treatments and interventions. We want to ensure the system is flexible and agile to facilitate timely access to treatments and improve equity of care for all Australians.

Current issues:

- There is **inadequacy of speed to market** in Australia when drugs are already available overseas
- The current process has no set time limits for commercial (pricing) negotiations which can lead to a protracted process **delaying cost effective access** to treatment
- No mechanism to enable **compassionate access** for people who are critically ill

Proposed solutions:

a. Government to work collaboratively with international regulatory agencies and

pharmaceutical companies to **share the approval process** ensuring products are provided in a **timely** manner. **Embrace new research technologies** to streamline and speed up the assessment of safety, quality and efficacy.

b. Consider the German model of **immediate access following approval of a drug**, particularly with life-saving drugs. The PBAC process and negotiations can then run while consumers benefit from treatments. This could also provide valuable data as to uptake, gauged across the wider population.

WE NEED: A robust and trustworthy HTA process that is flexible and can adapt to advances in technology to ensure timely cost-effective access to drugs and treatment following safety and efficacy approval by the TGA.

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4. <u>Further strengthen the transparency of the process that makes new therapies available.</u>

In line with the National Medicines Policy review we seek to increase transparency and evaluation of the process. End to end transparency must be a core principle to ensure trust in the system that facilitates access to a vital aspect of the healthcare Australians need.

Current issues:

- **Consumers are not provided with support, education, or updates** throughout the process by either the sponsors or Government. There is a lack of clarity in general process and timing information for consumers, including an impenetrable government website, leading to an inability to manage consumer expectations.
- There is a **lack of sponsor transparency** with insufficient submission information available in the public domain including information regarding sponsor registration in Australia and their intent to seek reimbursement
- **Clinicians are not provided with education** about access for patients via the TGA
- Lack of clarity as to the level of collaboration with EMA and FDA regarding **international approval and reimbursement** contract negotiations.

<u>Proposed solutions:</u>

a. Review the current mechanisms to ensure consumers are provided **with support, education and updates throughout the process** including the provision of <u>clear and accessible</u> publicly available information on HTA processes and **improved sponsor transparency**
b. Establish a **communication and education process with clinicians**
c. Establish a public **report back on all clinical trials** funded and available in Australia including location and recruitment numbers

WE NEED: Those most directly impacted by the work of the TGA and PBAC to be engaged and informed by the provision of appropriate, clear, accessible publicly available information on HTA processes plus targeted updates throughout the process.

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ABOUT THE AUSTRALIAN PATIENT ADVOCACY ALLIANCE (APAA)

The APAA comprises more than 20 Australian Consumer Health Groups – collaboratively representing the voices of over 15 million Australians living with chronic conditions – to pursue policy change.

Together we are speaking with one voice on systemic solutions to improve access, break down barriers and drive innovation that improves health outcomes for Australians.

Our shared ambition is to improve the health and wellbeing of all Australians through a health system that puts the consumer at its heart; one that draws on the experience of Australians living with health conditions and embeds consumers at every step of the policy making process.

We stand for:

- A consumer-centric healthcare system that delivers timely and equitable access to the best treatment, care and support
- Health policies that are co-designed with consumers involved at every step of the health policy development process
- Systemic reform of health policies affecting all Australians with chronic conditions

THE AUSTRALIAN PATIENT ADVOCACY ALLIANCE COMPRISES:

Arthritis Australia | Huntington's NSW ACT | Bowel Cancer Australia | CanSpeak | Cancer Voices Australia | Cystic Fibrosis Australia | Dementia Australia | Diabetes Australia | Endometriosis Australia | Haemophilia Foundation Australia | Kidney Health Australia | Leukemia Foundation | Lung Foundation Australia | Lymphoma Australia | Macular Disease Foundation Australia | Melanoma Patients Australia | MS Australia | Ovarian Cancer Australia | Rare Cancers Australia | Rare Voices Australia | Save our Sons Duchenne Foundation | Spinal Muscular Atrophy Australia