Pharmaceutical Benefits Advisory Committee

Reply to: PBAC@health.gov.au

The Hon Mark Butler MP

Minister for Health and Aged Care PO Box 6022

House of Representatives Parliament House Canberra ACT 2600

Dear Minister,

Thank you for your letter of 8 July 2024 requesting the Pharmaceutical Benefits Advisory Committee’s (PBAC) consideration of the final report of the Health Technology Assessment (HTA) Policy and Methods Review and its initial high-level reflections on the recommendations.

The PBAC acknowledges the extensive work done by the Review’s Reference Committee to develop its report. The PBAC appreciates the comprehensive approach taken to obtaining input from all parts of the community who have a stake in, and deep understanding of, HTA including health consumers and consumer organisations, health professionals, industry, advisory bodies, and international counterparts.

The PBAC recognises that while the Review’s recommendations are detailed, significant further design and consultation would need to be undertaken to implement them. The PBAC will be eager to work in partnership with health consumers, health professionals, industry and governments on potential issues, prioritisation of reforms, and redesign challenges at the appropriate time.

Over the past decade, the PBAC has closely observed the acceleration in development and market approval of medicines and other health technologies. As consumer and industry representatives, doctors, health professionals, and health economists, members also have first-hand experience of these changes as they undertake their work in the Australian community. We view investments in research and development to improve health outcomes positively. We are especially enthusiastic when we see these investments deliver new medicines and technologies that everyone can be confident will improve outcomes for health consumers and enhance the wellbeing of the broader Australian community.

While this acceleration has increased “speed to market”, it has also introduced new challenges. Traditionally, all medicines underwent rigorous assessment through three phases of clinical trials before seeking registration or reimbursement. Recent reforms to regulatory requirements have enabled pharmaceutical companies to seek registration through the Therapeutic Goods Administration (TGA) and subsequent reimbursement on the Pharmaceutical Benefits Scheme (PBS) earlier than before.

This has created greater uncertainty about how effective, safe, and cost-effective new health technologies are – including how well they might perform in the community compared to existing care. As the HTA Review found, the evidence for many technologies today does not allow us to be as

confident as we could be in previous years about how well they might perform. Further, the speed of the process and high expectations for faster access also limits opportunities for engagement with the community and ensuring HTAs capture all people that may benefit and the outcomes that are most relevant to them. The PBAC’s work increasingly involves balancing reduced confidence in claimed benefits, higher costs, and demands for greater engagement and faster access to touted breakthroughs.

# Consideration of the Review

The PBAC is pleased to see that the Review was guided by the principles of Australia’s National Medicines Policy (NMP), particularly the essential role of person-centred care. The NMP is an important framework that helps all partners contribute to the goal of achieving the world’s best health, social and economic outcomes for all Australians through a highly supportive medicines policy environment.

The Review’s findings largely reflect the PBAC’s experience. This includes the finding that Australia has a world class HTA system and that it is the result of decades of effort and continuous adaptation and improvement to meet emerging challenges and changing expectations. The PBAC also agrees that there are many opportunities to improve the system to better meet the aims of the NMP.

The PBAC also appreciates there have been several recent inquiries and processes that have, or may soon make, recommendations for reform to Australia’s health technology assessment and funding arrangements. This includes the report of the Enhanced Consumer Engagement Framework co- design project, the Senate Standing Committee on Community Affairs inquiry into equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer, and the House of Representatives Standing Committee for Health, Aged Care and Sport’s *The New Frontier* report. The PBAC believes it will be important that the HTA reform process takes a holistic view of these reports and their recommendations, while ensuring consistency with the NMP.

# Reflections on the recommendations

Overall, the PBAC is supportive of the Review’s objectives – particularly its focus on NMP objectives of equitable access and improving engagement and partnership with those who have a stake in HTA processes and outcomes. For this early opportunity, the PBAC offers the following high-level reflections which it hopes will assist you and the Government develop its response.

*Implementing the recommendations will be a large and complex body of work requiring collaboration by all partners*

The PBAC notes that many of the Review’s recommendations will necessitate policy changes, some of which will have significant budget impacts and will require careful consideration. The PBAC also recognises substantial work will be needed to fully scope the impact of recommended reforms and consult with relevant stakeholders.

The sequencing of changes must be thoughtfully planned to avoid disrupting the HTA system and adversely affecting consumer access to health technologies. Pragmatic implementation will require incremental improvements rather than wholesale disruption of existing processes. Given the current workload assessing submissions for health technologies, the PBAC recognises that areas in the Department of Health and Aged Care responsible for HTA are already overstretched and recommends that additional resources are provided so that the feasibility of implementing the recommendations can be fully explored with stakeholders.

The PBAC also acknowledges that changes to the HTA system will necessitate communication and effective partnerships with health consumers, health professionals, industry and governments.

Shared accountability for engagement and delivery is crucial, especially given the complex environment in which HTA operates. The PBAC is committed to working in partnership with those who have a stake in HTA through the HTA reform process.

*Recommendations to provide more equitable access to under-represented groups*

The PBAC appreciates it has an important role in Australia’s ambition to eliminate the disparity in life outcomes that exists between Aboriginal and Torres Strait Islander people(s) and non-indigenous Australians. To that end, it strongly supports the Review’s objective of improving equity of access for Aboriginal and Torres Strait Islander people, and their engagement and representation in HTA processes. The PBAC agrees that the consideration of impact on Aboriginal and Torres Strait Islander people(s) should be formalised in both submissions and HTA committee decision making.

The PBAC also supports recommendations to improve equity of access for children - to address the impact of exclusion from clinical trials and approved indications. However, the PBAC would also like to bring to your attention that there are many additional populations that are excluded from trials and approved indications such as people living with rare cancers or rare variants of genetic conditions and pregnant people. The PBAC receives frequent correspondence from people who are unable to access treatment that is otherwise supported by clinical guidelines.

The PBAC agrees that engagement early in HTAs with those who have a stake in the outcome, including consumers, carers and healthcare professionals, is essential to ensuring that people that may benefit from a health technology, and outcomes that are relevant to them, are identified and considered in the HTA.

*Balancing speed with equity and engaging the community*

The PBAC notes the emphasis that the Review report places on timely access. The PBAC supports reforms to streamline processes and resolve issues more quickly that would enable medicines to be subsidised sooner. The PBAC also recognises there are opportunities to adopt more proportionate approaches to assessment so that effort is directed to where it delivers most benefit to health consumers and the Australian community.

Reforms aimed at improving time to access for some should not come at the expense of other NMP objectives including fairness, equitable access, and safe and judicious use of medicines. It is important not to prioritize speed over engaging with those who have a stake in the outcomes of HTAs, a well-considered understanding of risk, informed choice, and well-coordinated person- centred care. In the PBAC’s experience, there are also often substantial clinical and consumer concerns when subsidy for a medicine is limited to a highly selected sub-group within the broader clinical population expected to benefit from the medicine. There are also significant challenges categorising clinical needs and developing different standards for different groups in a way that is defensible and meets community expectations. The PBAC does consider broader societal and consumer perspectives as a component of usual HTA processes, and thus advises against making exceptions for some groups by funding products that have not received rigorous evaluation.

The PBAC also continues to consider that total financial impact and opportunity cost are essential considerations in HTA given the unmet needs for health, community, social and residential care services that persist in the Australian health system and are relevant to many of the therapeutic areas PBAC considers.

*Opportunities to improve equity of access and engagement*

The PBAC would therefore like to highlight several additional opportunities it considers would help address equity of access issues and are worthy of early discussions:

1. The PBAC sees that there is an opportunity to address current restrictions that limit access to some medicines. PBS listings are bound by TGA registered indications even where treatment for other indications is supported by clinical guidelines. The PBAC believes that new ways to address this long-standing issue could be identified to provide more equitable access to medicines, especially for disadvantaged people such as children and adults with rare diseases.
2. The PBAC notes that many issues raised in the HTA Review were the result of a lack of published information about its processes (such as how it considers consumer input) and items on its agendas (such as information about proposed populations, comparators and outcomes), and plain language explanations of its recommendations. The PBAC, the Government and the pharmaceutical industry all share responsibility for providing transparency about PBAC submissions. The PBAC believes publishing details about applications relevant to health consumers, and adopting of recently emerged technologies to help develop plain language content for publication, represent opportunities to improve transparency and enable earlier engagement in the current resources constrained environment.
3. There is an immediate opportunity to better configure the relationship between the Life Saving Drugs Program and PBS. As recommended by the HTA review, there is an opportunity to reorganise this relationship to ensure that access to medicines for individuals with ultra- rare diseases is supported without additional administrative or evaluative steps.
4. Finally, the PBAC considers that adjustments could be made to the timing of PBAC meetings and how it considers different types of submissions to optimise the PBAC’s schedule. This would allow prioritisation of consideration of medicines proportionate to need or complexity.

The PBAC would like to thank you again for seeking its consideration and reflections on the HTA Review report and recommendations. The PBAC believes that there are many worthwhile recommendations that would add value and efficiencies to Australia’s current HTA system and improve access for Australians. The PBAC looks forward to seeing these progressed and working in partnership with health consumers, health professionals, industry and governments to deliver them.

Sincerely



Robyn Ward Chair

Pharmaceutical Benefits Advisory Committee October 2024