Collated feedback from Australian Patient Advocacy Alliance (APAA) to Health Technology Assessment (HTA) Policy and Methods Review Consultation options paper.

Overview

The Options Paper presents a significant step forward in reform of the HTA and demonstrates that the Reference Committee for the Health Technology Assessment (HTA) Review has genuinely listened to, and considered, feedback from previous consultations.

The outcome we seek from this review is a structured way forward to achieve optimal, faster HTA processes consistent with the National Medicines Policy, the consumer consultation and co-design projects and the goals of the HTA review.

Key principles

As the detail on implementation, including risk identification and mitigation is fleshed out, the solutions should include stakeholder input and must be consistent with the principles that are important to patients:

- Access must be faster and more equitable, particularly for paediatric patients and rare diseases
- There is more transparency and a seat at the table for consumers

Things we'd like to see carried forward:

- Measures to increase transparency, communication and meaningful stakeholder involvement and engagement through the entire process, including at the clinical trial stage.
- Measures to improve time to listing through streamlining, including an expanded role for the Pharmaceutical Benefits Advisory Committee (PBAC), and sorting out delays and inequitable access across states and territories.

Risks to successful reform:

- Over reliance on industry sponsors. The process should have capacity for a non-commercial sponsor to drive a submission where the submission is not commercially viable for a company.
- Potential for reduction in parliamentary scrutiny/oversight
- Not including consumers in codesign activities across all frameworks, including engagement and values frameworks; triaging criteria; and horizon scanning.
- Failure to address equity of access across Australia for technologies that have joint Commonwealth and state funding and may currently fall between the two funding pathways. This is particularly relevant for emerging or sophisticated technologies such as Genomic testing / CAR-T. The National Health Agreement must be a priority for Federal and State/ Territory Governments to address these inequities.
- Potential for disinvestment from medicines that are benefiting patients, bearing in mind that for some patients, for example those living with autoimmune conditions, effective disease management can be trial and error in terms of which medicines work and have tolerable side effects.

1. Transparency, communication, and stakeholder involvement in HTA

1.1. Transparency and communication of HTA pathways, processes and decisions

It is the view of our members that transparency, communication and stakeholder engagement are not end goals but are enablers that, if effectively implemented, will support the achievement of many other elements.

Publishing **plain language summaries** enables transparency and equity of consumer access in the context of diversity in health literacy. Current PBAC Agenda listings do not provide sufficient information for consumers.

Improvements to the **HTA website** are crucial to this outcome as this is the fundamental communication mechanism around HTA processes and decisions. It is imperative that there is consumer input to the design of the website to increase usability and effective knowledge translation.

1.2 Consumer, clinician and other stakeholder engagement and consideration in HTA

APAA support the involvement of consumers, clinicians and other relevant stakeholders in the development of an **engagement framework**. Whilst we recognise the diversity of stakeholders relevant to the HTA process, the consumer must have true equity as a key stakeholder.

Sustainable and systemic **support to establish and embed consumer input** is essential. The burden should not be on consumer groups to engage in the process, they should be supported to do so.

Consideration of the **inclusion of an advocacy body or advocacy representative on the PBAC** in addition to a consumer would be of benefit as it is often difficult for a single consumer or two to represent all consumers. Advocacy bodies have a broader understanding of the consumer experience and can add value to the experience.

2. Health technology funding and assessment pathways

2.1 Streamlining and aligning HTA pathways and advisory committees –

Overarching goal: a staged approach (including short, medium and longer-term steps) to achieving a simplified (single entry) HTA gateway reflecting nationally consistent HTA approach.

APAA supports **expanding the role of PBAC** across a broader range of health technologies including codependent health technologies. This streamlined approach will be critical for genomics and cell-based therapies. Currently these emerging therapies are not included and have no clear funding pathway.

The APAA members are supportive of a **unified, national, HTA pathway** for all health technology evaluation. This will enable the process to draw on appropriate specialists for all advanced therapies and technologies seeking public funding and to recommend the appropriate funding pathway.

2.2 Proportionate appraisal pathways: Development of pathways to calibrate the level of appraisal required for HTA submissions to the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents.

APAA support streamlining a pathway for **cost-minimisation submissions** to avoid delay in gaining access. Additionally, we support **price negotiation earlier in the process**. The current situation where a positive PBAC recommendation does not result in access due to high, non-sustainable cost is disheartening for patients and clinicians and a huge waste of resources.

3. Methods for HTA for Australian Government Subsidy (technical methods)

3.1. Determination of the Population, intervention, Comparator, Outcome (comparator is also addressed under economic evaluation)

APAA support **updated guidance** to require the explicit consideration of health equity and priority populations for new treatments. These populations should specifically include patient populations that can no longer benefit from the comparator classes of drugs due to either contraindications or prior loss of response.

3.2 Clinical Evaluation Methods

Methods relating to the use of Real World Data and Real World Evidence in HTA:

Taking a more holistic assessment of the impacts of medicines to inform its cost effectiveness, rather than just baseline clinical outcomes based on the lowest common denominator is positive. **Real-World Evidence** as well as consumer experience and outcome measures as well as disease-related experience, including post market evidence, must be considered in decision-making processes. For example, the Canadian agency, CADTH allows for patient perspectives.

The reform should include policy and methods that define high quality evidence that is fit-for-purpose for assessment of technologies where randomised controlled trial (RCT) evidence is not suitable (i.e. rare disease therapies and precision medicine).

3.3. Economic evaluation

The **current lowest price comparator model** and subsequent price reductions is resulting in newer therapies, that may be first in class, not being brought to Australia, even when given a positive recommendation by the PBAC.

Australia risks being seen internationally as an unattractive place to bring new therapies or even involve in clinical trials of new therapies due to the increasingly unlikely possibility of a price that represents actual cost of development and manufacture. This is counter to the goal of increasing access for Australians to best therapies.

Development of guidelines to distinguish between the selection of comparator for submissions claiming superiority and to submissions claiming non-inferiority should make clear which comparator should be selected when there are multiple potential comparators.

Although the consultation document acknowledges that the current economic evaluation of value for money does not involve **estimation of indirect and non-health benefits**, it has focused on health outcomes of clinical efficacy and safety with little advice about how indirect

and ‘non-health’ benefits can be quantified. Additionally, it assumes a very narrow definition of ‘health outcomes’, focused on specific clinical endpoints, and which is not necessarily related to the patient experience. It would be valuable to reconsider the definition of ‘health outcomes.’

4. Health technology funding and purchasing approaches and managing uncertainty

4.1. Approaches to funding or purchasing new health technologies.

APAA support bridging funding coverage for earlier access to specific therapies such as those that seek to address High Unmet Clinical Need, in particular for paediatric access to medicines.