RVA HTA Consultation 2 Submission

Overview

- The Options Paper has many positives and demonstrates that the Reference Committee (the Committee) for the Health Technology Assessment (HTA) Review has genuinely listened to, and considered, feedback from previous consultations. RVA congratulates the Committee.

Priority Options for Implementation

Given the breadth, depth and complexity of the options, RVA would like to highlight the following as priorities for the rare disease community.

- That consumers are involved in the codesign of outputs such as frameworks, triaging and reporting mechanisms, in particular rare disease consumers who often experience different and significant challenges in timely and equitable access to the best available health technology.
- That the impact of changes is assessed against KPIs informed by NMP and changes are made if the options are not achieving their intended purpose – in particular around timeliness, equity and access innovative technologies.
- All options identified in Section1 Transparency, Community and Stakeholder engagement, including identifying items that can be implemented relatively quickly for immediate impact. (Options 1.1,1.2, 1.3, 1.4)
- Options that are designed to ensure complex, co-dependent HUCN/HATV technologies that require joint funding from the Commonwealth and the states are prioritised and fully address current barriers to timely, consistent and equitable access experience by patients are addressed. This includes ensuring that there are pathways for genomic technologies that currently fall between Commonwealth and state funding pathways (Options 1.4, 2.1,4.3, 5.2, 5.6)
- Development of an explicit Values Framework codesigned with consumers. (3.2)
- Options for reduced time to access such as measures to reduce uncertainty, provisional approval, managed entry options , and measures to reduce resubmissions are prioritised. (2.1 Pathways for drugs for ultra rare disease, expanded role of PBAC, 2.2 Proportionate Appraisal pathways -early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN, 3.1, 3.2 in particular use of non-randomised evidence, values framework and pharmacogenomic technologies, 3.3 valuing long term benefits, valuing overall. approaches for managing uncertainty, 5.1)
- Enhanced use of RWD/RWE in all aspects of HTA. (3.2,4.3)
- Systematic data collection.(4.3)

Strengths, Areas for Improvement and Gaps

In our review of the Options Paper, Rare Voices Australia (RVA) would like to highlight strengths; areas that require further strengthening; and critical gaps that remain.

- **The strengths of the Options Paper are:**
  - All aspects that relate to increased transparency, communication and stakeholder involvement.
  - Measures to promote and ensure early, informed and meaningful consumer engagement through the entire process, including at the clinical trial stage.
  - The range of measures across each option to improve time to listing, in particular for High Unmet Clinical Need (HUCN)/high added therapeutic value technologies.)
  - Reducing delays to listing through streamlining, harmonisation measures and an expanded role for the Pharmaceutical Benefits Advisory Committee (PBAC).
  - An explicit values framework that transparently includes broader value of technologies in HTA.

- **Areas that need further strengthening:**
  - Ensuring that all frameworks involve consumers in codesign activities, including engagement and values frameworks; triaging criteria; and horizon scanning.
  - Sustainable and systemic support for consumers, especially those from small patient populations, to engage with HTA processes. (e.g. a key role of the HTA Consumer Evidence and Engagement Unit.)
  - Ensuring that assessments of rare disease therapies are informed by appropriate expertise and value for money assessments that are fit-for-purpose (Life Saving Drugs Program (LSDP) criteria to be maintained in PBAC evaluations).
  - Policy and methods that defines 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).
  - Mechanisms to ensure equity of access across Australia for technologies that have joint Commonwealth and state funding.

- **Critical gaps that remain:**
  - Funding models to address technologies that currently fall between Commonwealth and state funding pathways.
  - Capacity for a non-commercial sponsor to drive a Submission where the Submission is not commercially viable for a company.
  - Explicit performance measures that align with National Medicine Policy (NMP) objectives.