RCA Policy Briefing

November 2024

Health Technology Assessment (HTA) Reform

1. What is HTA?

Health Technology Assessment (HTA) is a set of processes used to decide whether new treatments – like medicines, vaccines, and medical devices – should be subsidised on the **Pharmaceutical Benefits Scheme** (**PBS** – for medicines) or the **Medicare Benefits Scheme** (MBS – for services such as diagnostic tests and appointments). Most of the cost is then paid for by the Government, making the treatment more affordable for the people who need it. HTA evaluates if treatments are superior to the current avaliable therapy and represent good cost-benefit.

Australia's HTA system was established in 1993, a time when the Pharmaceutical Benefits Advisory Committee (PBAC) primarily reviewed less complex drugs and single-target vaccines. It is broadly acknowledged that the system has not evolved to keep pace with modern advancements in medicines, vaccines, biotherapeutics, and genomics.

2. What is the HTA Review?

The HTA Review was agreed upon in the 2022–2027 Strategic Agreement between the Commonwealth and Medicines Australia and was led by a Reference Committee appointed by the Minister for Health and Aged Care. Between October 2022 and May 2024, the Review looked at the current HTA processes and proposed **50 recommendations** to improve access, equity, and the ability to evaluate complex and emerging technologies in a timely manner.

The Review involved two public consultations; deep dive sessions with industry, patients, and government representatives; and commissioned expert papers on topics such as international HTA methods, economic evaluations, and using data from real patient experiences.

The final report, published in September 2024, made many suggestions for improvement, but the Government has not yet responded. (See **ANNEX 1** for a summary of the recommendations by strategic goals.)

Alongside the Review, the Enhance HTA report recommends changes to better include the consumer's voice in HTA. This would make HTA more patient-centred by improving communication, support, and early involvement of consumers in the process. It makes recommendations to bring consumer input into decision-making and create ongoing collaboration between consumers, industry, and government.

3. Why does HTA matter for people with rare and less common cancers?

HTA plays a vital role in deciding whether people with rare and less common cancers can access new treatments through government funding. Currently, the system has some major problems:

Long wait times: On average, it takes 466 days from the time a new medicine is registered in Australia until it is subsidised (Medicines Australia, 2022).

Strict evidence required: The current HTA processes demand a level of evidence that is very difficult to gather for diseases that have small patient populations, like rare cancers. While it is extremely important to have an evidence-based assessment process, a more flexible approach could help, such as accepting evidence from "basket" or "umbrella" trials (where medicines are tested across multiple patient subgroups under a single protocol).

Outdated assessment methods: Medicines are still assessed based on cancer type, even though many modern therapies target genetic biomarkers. This means the same medicine may be subsidised for someone with a common cancer (e.g., breast cancer) but not for a rare cancer (e.g., sarcoma), even though they have the same genetic target.

Separate assessment pathways: Medicines (PBS) and diagnostic tests (MBS) are assessed separately, so a treatment may be subsidised but the test needed to identify whether someone would benefit from the treatment may not be. These are called companion technologies as they work together. If one is subsidised but the other is not then patients will continue to miss out because of price.

Lack of transparency: The current HTA process doesn't include patient perspectives in a way that is transparent. This limits how much consumers can input into and understand the decision-making. The Enhance HTA report says that adding a structured way for consumers to give input would help make HTA decisions better reflect what patients need and experience, creating a more inclusive, fit-for-purpose system.

4. Will the Review change things for people with rare cancers?

The Review acknowledges the many issues Rare Cancers Australia has advocated for, for over a decade, which is an important step. We welcome the Report and its reflection of RCA's submissions.
If adopted, the recommendations could have a significant impact for people with rare and less common cancers. They could:

- enable quicker access to medicines for people with rare and less common cancers
- allow pan-tumour therapies to be approved based on genetics, not just the type of cancer, meaning thousands more patients would benefit from affordable medicines
- use real-world evidence to support decision-making
- amplify the consumer voice, bringing more transparency and inclusivity
- introduce a bridging fund to allow patients to access Therapeutic Goods Administration (TGA) listed treatments while they go through the HTA process.

However, these are not yet government policy. They are still just recommendations. An Implementation Group is being set up to consider the report.

Dave's Story

In 2020, Dave was diagnosed with Stage 4 Metastatic Oesophageal Cancer, at the age of 52. He was given only 12 months to live. His oncologist helped him get a compassionate supply of a TGA-approved immunotherapy drug that was not yet funded by the PBS for his cancer type (but was for other cancers with the same biomarker), allowing him to access the drug without the prohibitive cost.

In 2022, the drug was listed on the PBS for his cancer type, giving Dave and his family a sense of relief and stability. Two years later, Dave continues to exceed survival expectations, experiencing sustained improvements and quality of life far beyond the initial prognosis.

However, the PBS listing limits funding this drug to a two-year duration, a decision that reflects HTA's reliance on initial trial data and cost-effectiveness models without incorporating real-world patient outcomes. As Dave's continued response to treatment defies conventional statistics, his family now face the daunting reality of needing to pay $70,000 to keep him on this life saving drug.

> **Access to immunotherapy is a matter of life and death where no patient should be forced to choose between financial ruin or medication to stay alive."**
>
> Dave, living with Stage 4 Metastatic Oesophageal Cancer

Dave's story underscores the critical need for HTA reform to allow more adaptive policies that consider exceptional responders and long-term patient benefits. Through flexible funding pathways, HTA could incorporate real-world evidence and patient-centred approaches to extend access to life-extending treatments for patients like Dave, ensuring that Australians are not forced to choose between financial security and life-saving care.

5. What next?

RCA will keep advocating for a fairer, faster system that is affordable for patients. We are working with patients, clinicians, Government and stakeholders across the sector to make these important changes a reality. We want to see:

- **a formal Government response** to the HTA Review Report and commitment to adopt all recommendations
- **fast adoption of the recommendations**, including pan-tumour pathways, better use of real-world data, bridging funding, and increasing patient involvement in decision-making
- **prioritisation of the needs of people with rare and less common cancers**, who face clear inequity within the current HTA processes.

Without urgent action, people with rare cancers will continue to face unacceptable delays in accessing life-saving treatment.

ANNEX 1: RCA Analysis of the HTA Review

Rare Cancers Australia has organised the 50 recommendations of the HTA Review into the following themes:

1. Enhance equity and access – Develop frameworks to ensure equitable access to health technologies, especially for underrepresented populations such as Aboriginal and Torres Strait Islander people, children, and those with unmet clinical needs.

- #1: Creating a more equitable system for First Nations peoples
- #2: Providing equitable access to medicines for paediatric patients
- #14: Improving time to access life-saving drugs for rare diseases
- #44: Identifying therapeutic areas of high unmet clinical need
- #45: Identifying therapies to address areas of unmet need
- #46: Proactive pre-HTA processes for high unmet clinical need

2. Accelerate evaluation timelines – Streamline processes to reduce the time required to assess and approve new health technologies, ensuring faster access to life-saving treatments.

- #7: Streamlined pathway for cost-minimisation submissions
- #8: Therapies with high added therapeutic value pathway
- #9: Pathways for therapies with added therapeutic value
- #11: Proportionate appraisal pathway for vaccines
- #12: Proactive vaccine assessment pathway
- #15: Jointly owned performance targets

3. Refine assessment methods and pathways – Implement proportionate, fit-for-purpose HTA frameworks tailored to the complexity and risk associated with different technologies.

- #3: Overarching recommendations for HTA processes
- #4: Unified HTA pathway and committee approach
- #5: Triaging submissions
- #6: Expanding PBAC advisory role
- #34: Overarching principles for methods
- #35: Methods for non-randomised evidence
- #36: Methods for surrogate endpoints
- #37: Methods preferred by decision-makers
- #38: Methods for biomarker-targeted therapies

4. Strengthen data collection and utilisation – Develop real-world data systems to improve decision-making, including cross-jurisdictional data sharing and governance frameworks.

- #27: Governance of real-world data
- #28: Data infrastructure development
- #29: Inter-governmental data collaboration
- #30: Real-world data methods development
- #31: Data collection for uncertainty resolution

5. Improve stakeholder engagement – Establish clear guidelines for consumer and stakeholder engagement, with a focus on transparency and inclusivity.

- #22: Publishing plain language summaries
- #23: Improving the HTA website and dashboard
- #24: Stakeholder engagement framework
- #25: Consumer involvement in HTAs
- #26: Explicit qualitative values framework
- #32: PICO scoping framework
- #33: Methods for assessing consumer evidence

6. Address emerging challenges – Develop frameworks to anticipate and manage new and emerging health technologies, including gene therapies and high-cost treatments.

- #21: Incentivising antimicrobial development
- #40: Comparator selection
- #43: Environmental impact reporting

7. Revise pricing and funding mechanisms – Create flexible funding models that address the financial complexities of high-impact therapies, including innovative payment arrangements.

- #16: Alternative contract and funding mechanisms
- #17: Pricing offer framework
- #19: Managed entry agreements
- #20: Bridging funding program
- #39: Discount rate revision
- #41: Cost-minimisation submissions
- #42: Valuing and pricing

8. Enhance post-market review – Strengthen mechanisms for post-market evaluation to ensure ongoing review and continuous improvements to listed health technologies.

- #10: Alternative modelling for disease areas
- #13: Improved processes for co-funded therapies
- #18: Updated post-review framework

9. Strengthen HTA workforce and resources – Expand the HTA workforce and resources to meet increasing demands, ensuring that there is expertise to handle complex evaluations.

#49: HTA evaluation workforce development
#50: Supporting architecture resourcing

10. Proactive planning – Establish horizon scanning functions to identify and prepare for emerging technologies, with a focus on addressing high unmet clinical needs and supporting long-term planning.

- #47: Horizon scanning function
- #48: Continuous review and improvement mechanisms