Health Technology Assessment Policy and Methods Review Reference Committee

Communique – 25 August 2023 meeting

The Health Technology Assessment (HTA) Policy and Methods Review (Review) Reference Committee (Committee) met by video conference on 25 August 2023.

Representatives from the Centre for Health Economics Research and Evaluation (CHERE) and the Adelaide Health Technology Assessment (AHTA) team were invited to discuss outcomes of some of their work for the Review. The Committee also had a discussion with a range of previous members of HTA advisory committees, including Professor Jon Karnon, Emeritus Professor Lloyd Sansom, Dr Suzanne Hill, Professor Rosalie Viney, and Professor Kirsten Howard. Support staff from the Review Secretariat in the Department of Health and Aged Care (Department) attended.

What did the Committee discuss?

Presentation of paper HTA Methods: Economic evaluation

Research leads from CHERE provided the Committee with a briefing on the early draft of their paper on HTA Methods: Economic Evaluation. The briefing included an overview of the methodology used to source and assess information for the paper to date and initial findings.

The Committee heard that as is the case in Australia, Cost Minimisation Analysis (CMA) is accepted as the main economic approach used where a treatment does not claim to have a substantial improvement in efficacy or reduction in toxicity compared to alternatives, across many of the countries analysed. The Committee heard that some countries including England and Singapore state that CMA is a faster process compared with treatments that are claiming a substantial improvement in efficacy or reduction in toxicity. The Committee heard that Reference pricing is used across many of the studied countries. However, there are some differences in the basis of the reference price for some countries. For treatments that do claim a substantial improvement in efficacy or reduction in toxicity compared to alternatives, the Committee heard that Cost-Utility Analysis Cost-Effectiveness Analysis are the preferred approaches for most countries studied as with Australia. The Committee heard that they found four countries where the HTA authority used explicit thresholds including England and Wales where there is specific guidance around a range of maximum acceptable Incremental Cost-Effectiveness Ratios .Similarly, Norway, the Netherlands, and Sweden all apply explicit thresholds, with quality-adjusted life year weights applied in Norway and the Netherlands while Sweden applies a weighting based on a qualitative assessment of disease severity. The Committee heard about the different methodology for applying weighting in different countries. The Committee requested that the international examples be contextualised for the Australian setting, including a potential future case study of an individual drug's listing and its HTA processes that have been implemented for the same

listing across different jurisdictions. The Committee noted the variability of recommended patient reported outcome instruments for economic evaluation. The Committee noted the variability in inclusion of indirect and non-health benefits by HTA agencies, specifically its conditional use in supplemental analyses by every jurisdiction studied except for the Netherlands, which included this societal perspective in its base case.

There was discussion regarding the methods of extrapolation and discounting across jurisdictions. The variability in guidance relating to time horizon within different jurisdictions was noted.

The Committee heard the different methodologies of assessing and managing economic uncertainty, while noting the differences in preferred analysis methods within mandatory and recommended guidelines of respective jurisdictions. Methods discussed included deterministic sensitivity analysis and probabilistic sensitivity analysis which both aimed to address structural and parametric uncertainty arising from limitation in the availability and quality of supporting evidence.

In the context of special consideration of rare diseases, high unmet clinical need, and disadvantaged populations, the Committee discussed models from other jurisdictions and their applicability to the process used by Australia noting that many processes were linked to unique features of those jurisdictions, making direct comparisons challenging. The Committee was advised that Australian guidelines are consistent with international standards and allow for flexibility where appropriate. The Committee identified areas for CHERE to conduct further inquiries.

Presentation of Consultation 1 Report

The Committee was presented with an updated presentation of the report being prepared by CHERE to summarise and synthesise the stakeholder submissions made through Consultation 1 for the HTA Review. The Committee heard that the report is proposing to summarise the submissions in line with nine major identified themes. Additionally, the report details submissions made by different stakeholder groups including individuals and organisations representing patients, industry, research, academia, consulting, and government departments. The Committee discussed the accuracy and currency of some of the information and noted that the report was a summary of a broad range of views and information presented by stakeholders rather than an analysis of the views expressed. The Committee discussed some of the recommendations made by stakeholders in their submissions, including their potential practicality and implications. The Committee identified areas where CHERE was asked to clarify and areas for additional analysis.

Experiences of previous HTA committee members

The Committee heard from a range of previous members of HTA advisory committees regarding their experiences. The Committee noted that the HTA system in Australia is widely considered to be world class, however this does not mean that there are no further improvements that could be achieved. A range of issues were discussed including the increasing pace of scientific development which results in a shorter period where a therapy has the comparative advantage before another treatment replaces it, which incentivises the product developers to seek to expedite time to market. The Committee heard about models used in different international jurisdictions to reduce time to market. The Committee discussed some of the limitations in the Australian context for expediting treatments to market including that companies have discretion of when to apply to supply their product in

Australia, noting that companies often do not make applications in Australia for several years after their applications to international jurisdictions for a range of reasons. The Committee also noted that this may be partly due to the population size of Australia making it less of a priority for industry. The Committee heard that there is a trend for companies to be making applications earlier in the development processes which creates an increased priority on how to manage uncertainty. There was a discussion regarding strategies that could be used to incentivise companies to make applications to the Pharmaceutical Benefits Advisory Committee (PBAC) earlier.

There was discussion around the propensity for multiple submissions as part of sponsors' process for refining their submission, and the inefficiency that this creates. The Committee noted that being able to solve some of the crucial issues within submissions before they get to the PBAC could have substantial benefits to timing and process. The Committee discussed what options may exist that could allow for early (pre PBAC) advice on submissions and some of the associated practicalities and issues. The Committee considered a range of potential opportunities to streamline some of the processes in the HTA pathway to enable faster access.

The Committee considered how to manage clinical uncertainty for treatments for conditions with high unmet clinical need where there is limited data, including the possibility of managed entry schemes to enable evidence development.

The group noted the value in increasing and bringing forward consumer input into the HTA process acknowledging the valuable work being undertaken by the Consumer Evidence and Engagement Unit and the Deputy Chair of the PBAC, Jo Watson. The Committee emphasised that one integral element to being able to speed up time to access and improve HTA processes generally is the capacity and capability within the system including the resources and staffing within the Department.

International health technology market approval, funding, and assessment pathways

The Committee received a presentation from AHTA on further development in its analysis of options around HTA systems. Relating to pathways for registration and reimbursement of health technologies internationally, the Committee heard that of the jurisdictions included in the analysis, there were 16 jurisdictions with pooled, multi-payer healthcare systems, where multiple entities (such as government/social insurance organisations or private insurance companies) pay for healthcare services, and 11 jurisdictions have single payer systems, where a single entity (such as the government) pays for all healthcare services for their citizens. Additionally, 17 of the 27 jurisdictions reported conducting HTA reactively, however some jurisdictions (4 noted) have proactive HTA mechanisms with a further 6 having both proactive and reactive.

The Committee heard that as with Australia, HTA is generally conducted internally to government or by national independent HTA agencies with a relationship to government.

HTA is conducted reactively (i.e., submission received and assessed) to inform the reimbursement of medicines in most of the jurisdictions reporting on this (17/27). The Committee heard that the average time from submission of the evidence dossier by sponsors to HTA funding recommendation was 17 – 26 weeks with some countries taking 60 – 72

weeks. There is large variation in the timeline for patients to have access to funded medicines with few countries having a specific timeline for listing the drug after the HTA recommendations. Additionally, many countries did not report on the timelines entirely.

The Committee heard that there were 8 jurisdictions that allow early access and prioritisation of certain medicines based on pre-determined criteria. The prioritisation mainly occurs through topic selection (proactive HTA) and expedited reviews for medicines that fulfil the criteria of high unmet clinical need.

The research leads at AHTA informed the Committee that very few countries have a consistent process to disinvest low-value or obsolete medicines, instead relying on market (use in clinical practice) resulting in sponsor withdrawal. Further, for countries where there is a process, it is mainly price readjustment through periodic re-assessment of the listed medicines.

Regarding the flexibility in the HTA systems and decision making, the Committee heard that other jurisdictions, including Austria, Canada, France, Japan, Switzerland, UK, were not limited to positive and negative recommendations, instead allowing conditional reimbursement based on clinical need. Similarly, 10 jurisdictions mentioned having flexible HTA outcomes for a specific use of medicines such as for rare diseases with different jurisdictions applying different criteria, such as satisfying high unmet clinical need, orphan drugs, medicines with uncertain long-term benefits. However, due to small patient population, uncertain long-term effects, considerable budget impact and other issues, specific medicines may be conditionally reimbursed with the requirement of further evidence development with the consequence of failing to meet the requirements of reimbursement potentially resulting in delisting or disinvestment, depending on the agreement.

On the topic of transparency of HTA processes and decisions, AHTA informed the Committee that most jurisdictions have fully or partially transparent HTA process. With most HTA bodies producing a summary of the submission that is published alongside an account of the assessment and the HTA funding recommendations. However, the amount of information and the proportion of redaction in documents differ across countries.

The Committee heard that while 13 of the 27 studied international jurisdictions have a parallel regulatory and HTA approval process, only 4 of those is for all medicines, with the other 9 only relating to specific types of medicines.

The research leads at AHTA informed the Committee that stakeholder engagement is prominent in HTA with the stakeholder most consistently engaged being industry, followed by clinicians, and then patients. Of note, roughly a third of the scholarly literature found focused on patient engagement, indicating this topic has been prominent in the past 10 years. Further, leading jurisdictions have dedicated patient engagement staff or committees, whose activities include conducting original qualitative research with patients, and they profess to use information from patients in all phases of the HTA pathway.

The Committee heard about the use of special HTA pathways used internationally including for technologies for rare diseases or for small patient sub-populations/ultra-rare mutations, populations for which there is a high unmet clinical need, vulnerable and/or disadvantaged patient populations, technologies with uncertain long-term outcomes, co-dependent technologies, antimicrobials, new ‘advanced’, high-cost therapies (e.g. cell and gene therapies), and technologies/indications where there is no current sponsor/application (e.g. repurposing of listed medicines or unlisted medicines for very small populations).

The Committee heard that most jurisdictions studied included a consideration for equity however there were few programmatic or methodical approaches to integrating equity considerations into assessment or appraisal reported.

The Committee heard about the implication of AHTA's findings for the Australian setting including opportunities for optimisation of the current HTA systems and processes to improve timeliness and transparency.

The Committee identified areas where AHTA was asked to conduct further inquiries and undertake further analysis.

Meeting close and next meeting

The Committee noted the next meeting will be held on 7 September 2023.