Response to HTA draft Terms of Reference

Submitted via survey on consultation website:
https://ohta-consultations.health.gov.au/ohta/cd36070a/

1. Do you foresee any challenges in addressing the issues listed for consideration by the HTA Review (per Clause 5.3.2 of the Strategic Agreement)? Which issue(s) and what are the challenges?

Challenges/issues - or Nil input? (Please aim to keep the response under 500 words)

"Clause 5.3.2 of the Strategic Agreement states that, without limiting the matters the Reference Committee considers important to review, the HTA Review will address the following issues:

1. *selection of comparator(s)*
2. *methods for evaluating rare diseases for reimbursement and alternative funding pathways if required*
3. *methods for evaluating new and emerging technologies (including cell and gene therapies, and other precision based medicines) and the suitability of existing funding pathways as required*
4. *methods for evaluating all new medicines and vaccines*
5. *use of real world evidence for evaluation including use of evidence from sources other than randomised controlled trials;*
6. *managing clinical, economic, financial and other uncertainty*
7. *examining the feasibility of international work sharing for reimbursement submissions."*

At a general level, when addressing the seven issues, the Reference Committee will need to navigate the significant scope of the Review and articulate the rationale behind what is (and is not) emphasised in the final report. When addressing each issue and prioritising content for inclusion, consumer needs cannot be an afterthought – the Committee will also need to be cognisant of the different challenges across those issues for different diseases, and of underserved populations.

More specifically, an important challenge the Reference Committee will face in addressing issues 2, 3 and 4 in a way applicable to blood cancers is that our understanding of blood cancer disease subtypes is becoming increasingly more defined, and patient populations of those diseases are subsequently smaller and more refined. While this is of benefit to patients, allowing for the development of more targeted treatments and precision medicine, it also creates challenges and barriers for patient access to new treatments and diagnostics.

This is particularly acute with respect to evidence development and public funding for novel therapies (both medicines and diagnostics) and compounded for new therapies that require a mix of state and federal funding (for example, CAR T-cell therapies). The regulatory and reimbursement system is adjusting to the advent of precision medicine which, while increasing our understanding of blood cancers and their treatments, has brought new challenges for traditional models of development, regulatory approval and reimbursement, as well as challenges to ensuring patients have access to best practice diagnosis, treatment and care.

The Leukaemia Foundation is able to provide further advice throughout the course of the Review on the unique challenges associated with evaluating rare blood cancers for reimbursement/alternative funding pathways and evaluating new blood cancer treatments.

2. Are there any HTA policy and methods issues that do not fall within the areas identified in the draft ToR that you think should be included? What are they? And why should they be included?

Other issues - or Nil input? (Please aim to keep the response under 500 words)

A key challenge for the Review will be one of emphasis, and in this context, we submit that the final version of the ToR should commit the Reference Committee to more explicitly:

1. Examine how HTA can better recognise the importance of equitable access.

Consultations and evidence gathered in developing the National Strategic Action Plan for Blood Cancer found that two of the key risks to patient access were 1) inequitable access to novel and specialised therapies by jurisdiction due to the high cost of therapies and complex funding arrangements, and 2) equity of access risks to existing and emerging therapies, especially cellular therapies.

The draft Review ToR includes "balancing equity and efficiency in HTA" and "equitable distribution and efficient use of limited HTA resources." However, the large number of items to be covered, and the permanent caveats around efficiency, potentially undersell the access challenges faced by many patients and the importance to patients and our health system of improving these.

2. Include a focus on genomics and cell and gene therapies.

While the draft ToR includes "highly specialised therapies (such as cell and gene therapies)" as one of the health technologies to be considered by the Review, this language is dilutive, especially when coupled with the large number of other items within the ToR's scope.

The ToR should more explicitly state these therapies are a focus, which would be commensurate with their potential future impact for patients and on our health system. This extends to genetic and genomic testing, which is increasingly recognised as part of routine care in other markets comparable to Australia.

3. Identify where proposed solutions could potentially be applied to HTAs for medical services.

The Review is not considering HTA policy and methods outside of industries not party to the Strategic Agreement (devices, digital technologies and medical services), but it is important that the links are explicitly made to avoid duplication and/or further complicating an already complicated system.

As such, while we acknowledge the ToR includes 'implications of any recommendations for assessment of other health technologies and hospital funding' (section 4.3), we recommend that the ToR builds on this to require the Reference Committee to clearly identify and discuss which of its recommendations (e.g. on transparency) may be applied to HTA processes for other medical

technologies (beyond just possible implications), and identify the steps Government should take to achieve this.

4. Canvass the importance of patient engagement improvements.

The separate process to elevate the patient and consumer voice (agreed between the Commonwealth and Medicines Australia in the Strategic Agreement) should not result in scoping out "Processes for patient and consumer engagement" in their entirety from this HTA Review (section 5.1).

The draft ToR includes "incorporation and use of direct input from patients" (section 4.2) within scope. We urge the Committee to retain this reference in the final ToR, and to at a minimum add a note referencing to the criticality of patient input to the HTA process and strengthening the commitment to harmonise with the separate consumer process. Not doing so risks diminishing the importance of improving the HTA patient engagement experience, and by extension, the quality of those processes.

Finally, we acknowledge the complexity of the task faced by the Reference Committee given the complexity of the system it is reviewing. We stand ready to assist and contribute when and as needed. Accessing new treatments is integral to saving lives from blood cancers, and we look forward to constructively engaging with the process throughout the year.