23 February 2024

Reference Committee
Health Technology Assessment Policy and Methods Review
Department of Health and Aged Care
GPO Box 9848
Canberra ACT 2601

Via email: htareviewconsult@health.gov.au

Dear Reference Committee

Re: HTA Review Consultation 2

Thank you for the opportunity to provide a submission to the *Health Technology Assessment Policy and Methods Review – Consultation 2.*

The Leukaemia Foundation is the only national organisation representing all Australians with blood cancer. For over 45 years we have provided a variety of supports and services, and funded life-changing research. We provide evidence-based policy advice and amplify the voices of those affected by blood cancer.

Access to therapies is critical to blood cancer patients. As such, we have engaged in this Review process from its inception. This has included contributing to:

- The Terms of Reference consultation
- Consultation 1
- Appearing before the Reference Committee as part of a 'Deep Dive' session into our proposed Right to Trial program
- Attending the online workshop held 15 February as part of Consultation 2.

We have articulated a clear and consistent agenda for change throughout these engagements.

This submission articulates the key points we feel the Committee needs to consider from a blood cancer perspective, as they relate to the main components of the comprehensive set of options outlined by the Committee in the options paper.

We thank the Committee for its work on the Review, and for its consideration of the issues highlighted in this submission. We would be happy to discuss further and can be contacted at ctanti@leukaemia.org.au.

Sincerely,

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Chief Executive Officer

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SUMMARY OF ISSUES AND PROPOSED SOLUTIONS

We are committed to ensuring that the perspectives and needs of patients with blood cancers are front and centre in the Health Technology Assessment (HTA) process. We provide the following general, high-level feedback on the variety of options proposed in the Options Paper, which focus on ensuring the needs and voices of patients with blood cancers are heard and prioritised in the HTA processes.

Throughout the HTA review process, we have highlighted a series of challenges that have included:

- Current evidence requirements – particularly given that uncertainty in evidence increases where there are fewer patients and many blood cancers are rare, and that rapid advances in genomic profiling are enabling even more accurate subtype diagnosis.
- Greater responsiveness to newer therapies is required
- System fragmentation, cost shifting and increasing funding complexity – particularly across government lines of responsibility and particularly for highly specialised therapies
- An access 'postcode lottery' exists, particularly given costs to individuals and availability of clinical trials
- System costs (especially for newer therapies)
- Opaque processes and difficult-to-understand HTA procedures (including lack of transparency; difficult-to-access information; limited stakeholder involvement; and lack of clear decision criteria)
- Insufficient of consideration of Patient Reported Outcomes in HTA.

To help address these challenges, we proposed the following solutions:

- Right to Trial - to support systematic evidence development and provide a mechanism for the more systematic evaluation of off-label use and re-purposing of drugs.
- Additional reimbursement pathways - allow for the use of earlier and more varied clinical data, including international data such as through Project Orbis; rolling review of confirmatory clinical trials or real-world data; and increasing use of international data and real-world evidence.
- A harmonised, national approach to cellular and genetic therapies.
- Listening to consumers and generating more evidence for HTA – The National Institute for Health and Care Excellence (NICE) in the UK has also made revisions to its HTA processes to better capture consumer views in HTA processes.

The commentary we have provided below on the Options Paper draws from these previously expressed challenges and solutions, and applies them to the specific options in the paper.

COMMENTARY ON THE OPTIONS PAPER

General

Visibility in the HTA process has traditionally been a significant issue, with patients and patient groups often feeling sidelined.

It is important that the HTA process incorporates a more engaging approach to consumer involvement. This engagement should be interactive, allowing patient groups to be active participants in discussions rather than passive recipients of decisions. Such involvement is essential throughout the entire process of listing therapies, ensuring that patient perspectives are considered at every stage.

The relationships and processes between Federal and State and Territory Governments require streamlining to improve efficiency. The current lack of coordination and data sharing between these levels of government creates barriers to access for patients, who are consequently denied timely access to essential therapies. Addressing these issues is paramount for improving patient outcomes.

The differing cycles between the Pharmaceutical Benefits Advisory Committee (PBAC) and the Medical Services Advisory Committee (MSAC) further exacerbate these barriers, leading to delays in the listing process for new therapies.

Timely access is important to patients, and the current system does not always facilitate this – particularly when access to therapies is funded across different levels of government. As the *National Health Reform Agreement (NHRA) Long-term Health Reforms Roadmap* acknowledges, the current HTA approach "does not support coordinated and timely responses to rapidly changing, emerging, and disruptive technologies, including high-cost and highly specialised therapies and services."

We ask that as the Reference Committee finalises its report, these principles remain front-of-mind and feature in the final report.

Transparency, communication, and stakeholder involvement in HTA

The intent to improve the information available regarding HTA systems, processes, pathways, and decisions is welcome.

Significant improvements to the website are required. Relevant documents, reports, and data related to HTA processes are often not easily accessible to the public (inclusive of complex terminology), making it difficult to gain a comprehensive understanding of the assessment process.

The dashboard may be useful, but it would not be enough to 'communicate the status of health technologies moving through the HTA system' – those different elements of the system need to be defined, in plain language, and the specific actions consumers can undertake needs to be articulated on the dashboard. Otherwise, it becomes further information for consumer to process, without the benefit being clear.

One element that could be stronger in the options paper is the need to improve navigation, and for consumers to be able to find consumer information (specifically for non-sponsors).

We support adding additional guidance to the PBAC guidelines on RWE and PROMs, on the condition this is developed with consumers. Further, the *National Strategic Action Plan for Blood Cancers* called for a national system for patient reported outcomes in blood cancer, and in that context we support consistency in the implementation of RWE/PROMs across HTA systems and processes.

We also support:

- The increased focused on Patient Reported Outcomes (PROs) in the options paper. We had called for greater use of PROs in HTA, as they are not adequately used in current HTA processes.
- Attempts to increase collaboration between the Federal Government and State and Territory Governments. In this context, the options regarding centralised data sharing and data standardisation, work sharing, and nationally cohesive approach to HTA as outlined in Schedule C of the *2020-25 National Health Reform Agreement Addendum*, are welcomed.
- Collaboration among the Federal and State and Territory Governments to ensure a coordinated national approach to supporting the development of cellular and genetic therapies. There is still further work required in particular on ensuring collaboration on newer, high-cost therapies.

Health technology funding and assessment pathways

As a patient representative organisation, we do not have a specific view on the optimal price negotiation approach. However, we encourage the principles of early patient access and affordability to patients to be at the forefront of this decision-making process, and that these are not used to justify price negotiation tactics.

The paper notes that 'As the development of disease- specific models would require significant investment to develop, they would only be used for disease areas where many subsequent submissions would utilise the model.' This is restricting for rarer diseases, and also may be challenged in time as genomic profiling allows better identification of different types of diseases and their sub-types.

We do welcome the options paper's attempts to embed patient involvement right throughout HTA processes, including around Therapeutic Goods Administration involvement.

Methods for HTA for Australian Government Subsidy (technical methods)

We reiterate the findings in the report that HTA assessments should allow more flexibility in the evidence base, including greater acceptance of non-randomised evidence and the role of RWD, and the lack of information about how elements (beyond clinical effectiveness, cost effectiveness and financial impact) such as patient and consumer input were being considered in HTA decision making.

We are supportive in particular of the options regarding updated guidance to require the explicit consideration of health equity and priority populations for new treatments, and methods for the assessment of nonrandomised and observational evidence (including RWD/RWE).

We have advocated for the earlier input of consumers. In that context, we support earlier involvement in PICO as outlined in the options paper:

- "Increased early input on the PICO from patient and clinician communities to ensure all relevant patient populations that could potentially benefit from the new therapy are considered in the HTA, and to identify issues that may impact implementation early to be addressed (for new drugs or major expanded indications claiming added therapeutic value)."

It will be, of course, important to try to ensure the impact on timeframes is minimal.

Health technology funding and purchasing approaches and managing uncertainty

The proposal around 'time-limited funding for therapies that may address areas of high unmet clinical need (HUCN) where there is significant clinical, economic and financial uncertainty' is similar to the Leukaemia Foundation's proposed 'Right to Trial' program.

This initiative seeks to provide patients with access to potentially life-saving treatments in the absence of comprehensive long-term data. It proposes a systematic approach to evidence development, offering a pathway for the evaluation of off-label use and the repurposing of drugs.

Right to Trial (which is different to the "Right to Try" program overseas) aims to bridge the gap in access to potentially life-saving treatments where traditional evidence requirements are challenging to meet.

We support approaches that aim to balance the need for rigorous assessment with the urgent needs of patients facing limited treatment options.

The options presented relating to reforms to optimise access to and use of RWD in HTA, and guidance on the use of RWD and RWE, are both also welcomed.

Futureproofing systems and processes

We agree with the findings that Australia does not proactively assess unmet clinical need, and that gaps in work-sharing locally and internationally are important.

The associated options relating to a priority list of areas of HUCN to be developed and regularly reviewed, and to improving HTA capacity and workforce in Australia, are needed but will need consultation and transparency as these are developed and updated in time.

Final comments

There are potentially wide-reaching impacts regarding the proposed 'unified, national, HTA pathway for all health technology evaluation.'

Some of these may only become apparent once this is fleshed out, and once implemented. There are benefits, primarily around the possibility of faster, streamlined processes without having to deal with the variances across different processes that currently exist – but there are risks, particularly given this proposal is coming predominantly from a medicines-focused review.

Similarly, it is also unclear what exactly is being proposed for the Life Saving Drugs Program.

The options, and how they are implemented, should also be dependent on the contents of the finalised consumer engagement framework.

As with all large-scale reforms, implementation is important and will to some extent determine the success or otherwise of these proposed initiatives.

The Leukaemia Foundation remains supportive of the work of the Reference Committee, and we look forward to continuing to help shape and implement the finalised reforms.