

16 June 2023  
Rosemary Huxtable PSM  
Michael Walsh PSM  
Independent Reviewers  
Mid-Term Review of the NHRA Addendum 2020-2025

Via email: [NHRAReviewSubmissions@health.gov.au](mailto:NHRAReviewSubmissions@health.gov.au)

Dear Independent Reviewers

**Re: Mid-Term Review of the National Health Reform Agreement (NHRA) Addendum 2020-2025**

Thank you for the opportunity to provide a submission to the *Mid-Term Review of the National Health Reform Agreement (NHRA) Addendum 2020-2025*.

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.

Healthcare funding arrangements, including those between the Commonwealth and the States and Territories, impact patients because they heavily influence what services patients can access, where they can access them, and at what cost.

We welcome this Review of the NHRA. While we acknowledge that the NHRA is a funding agreement between the Commonwealth and the States and Territories, the NHRA needs to result in better support for patients through fixing access gaps that manifest due to ongoing system fragmentation present in current funding arrangements and associated system design.

Specifically, this submission suggests:

1. Recognition that patients are both funders and recipients of healthcare
2. A harmonised, national approach to cellular and gene therapies
3. Elevate Patient Reported Outcomes
4. Examine improving stem cell transplant and donation funding arrangements

Thank you for your consideration of the issues highlighted in this submission. We would be happy to discuss further and can be contacted at [ctanti@leukaemia.org.au](mailto:ctanti@leukaemia.org.au).

Sincerely,



**Chris Tanti**  
Chief Executive Officer

## ***About the Leukaemia Foundation***

The Leukaemia Foundation is the only national organisation that represents all Australians living with blood cancer – including leukaemia, lymphoma, myeloma, myeloproliferative neoplasms (MPN), myelodysplastic syndromes (MDS) and amyloidosis.

We provide the following free services to patients:

- Personalised information and support from highly trained Blood Cancer Support Coordinators for patients and their loved ones alongside a range of health and wellbeing services
- Accommodation near major hospitals around Australia and help getting to and from the many appointments that come with a blood cancer diagnosis
- Trusted information to empower people to navigate the road ahead, including critical education, support groups, booklets, newsletters, and online information

The Leukaemia Foundation's research program drives rapid advancements in blood cancer treatments, encourages the careers of promising scientists, and helps give Australians access to global clinical trials.

## **Context: The imperative to improve blood cancer outcomes**

This year 19,403 Australians will be newly diagnosed with a blood cancer. This is equivalent to 53 people every day or one person every 27 minutes.

Over the past 10 years, the **incidence of blood cancer has increased by 47%**, and 135,000 Australians are now living with a blood cancer. Sixteen Australians will lose their life to blood cancer each day and 1 in 3 people diagnosed with a blood cancer will not survive five years after their diagnosis.

Blood cancers require, in many instances, highly specialised and complex care throughout the treatment period. Many blood cancers can have repeated acute episodes of treatments, remission and relapse.

Some blood cancers can become refractory to current treatments, leaving patients with very limited options. Treatments are often aggressive, highly toxic and can result in debilitating lifelong side effects.

The health and social imperatives for action are underlined by an economic imperative. By 2035, **blood cancer will cost the economy \$71.9 billion each year**. Myeloma and leukaemia are the first and third most expensive cancers for our health system to treat.<sup>1</sup> Around 43% of individuals report out-of-pocket expenses, and more than one in three of these incur more than \$A5,000 in cost.

Using the latest available cancer registry data, the *State of the Nation: Blood Cancers in Australia 2023* report showed that if best practice clinical treatment and care for blood cancers were consistently implemented, potentially up to 29 per cent of blood cancer mortality could be prevented.

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<sup>1</sup> Merollini, K.M.D., Gordon L.G., Ho, Y.M., et al., 2022, Cancer Survivors' Long-Term Health Service Costs in Queensland, Australia: Results of a Population-Level Data Linkage Study (Cos-Q), *Int J Environ Res Public Health*, 19(15), 9473, doi: 10.3390/ijerph19159473.

However, this on its own will not address blood cancer mortality, especially for blood cancers with the poorest prognosis. Consequently, significant improvements in survival for people living with blood cancer cannot be realised without new investment in research into new therapies and care.

## 1. Recognition that patients are both funders and recipients of healthcare

The Leukaemia Foundation reiterates the importance of including patients' needs and perspectives front and centre of this – and other – funding agreements.

We acknowledge the NHRA is an agreement between the Commonwealth and the States and Territories, and that it does also not focus on health system policy broadly.

Nonetheless, all parts of our healthcare system, and associated funding arrangements, need to support the fundamental right of patients and taxpayers to have access to medicines and healthcare interventions funded by taxpayers.

Taxpayers fulfill their end of the social contract in healthcare by paying taxes. The government, as the custodian of public funds, has the responsibility to allocate tax revenues efficiently and effectively.

Further, patient-centricity in healthcare policy, practice and funding is becoming increasingly entrenched and mandated. Recent examples include:

- The National Medicines Policy (NMP) – "The NMP acknowledges the fundamental role of consumers in achieving the policy aim by placing the individual at the centre, and by focusing on and responding to the needs of Australia's diverse population."<sup>2</sup>
- The \$2.2 billion package of measures announced (28 April) at National Cabinet, where National Cabinet articulated their shared vision for 'a patient-centred and sustainable Australian healthcare system.'<sup>3</sup>

The current Addendum "recognises that responsibility for health is shared between the Commonwealth and the States, and that all governments have a responsibility to ensure that systems work together effectively and efficiently to produce the best outcomes for people."

To truly fulfil this 'responsibility' for systems to work together effectively, patients – and their definitions of "the best outcomes" (see section 3) – need to be reflected in the NHRA.

The LF reinforces that the centrality of patients' needs and perspectives should be integral to both policy frameworks and funding arrangements. This needs to be given greater prominence in the next iteration of the NHRA.

### RECOMMENDATION

- That the Review highlights the centrality of patients as both the recipients of care as well as the primary funders of government services.

<sup>2</sup> *National Medicines Policy*, <https://www.health.gov.au/sites/default/files/2022-12/national-medicines-policy.pdf>

<sup>3</sup> *National Cabinet Statement*, <https://www.health.gov.au/news/national-cabinet-statement-on-a-better-future-for-the-federation>

## 2. A harmonised, national approach to cellular and gene therapies

### *The 'postcode lottery'*

There remains inconsistent access to diagnostics and novel and specialised therapies across Australia – leading to a 'postcode lottery' of blood cancer outcomes. Research demonstrates that this contributes to potentially preventable loss of life, poor quality of life, and inequitable outcomes across patient groups.<sup>4</sup>

Many newer diagnostics, treatments and therapies are available only through either clinical trials (which are mostly available at major metropolitan treatment centres) or at the patient's own expense. This can limit access to people in regional/remote areas or those who cannot afford to access non-PBS treatments. The pathway to PBS reimbursement for some of these therapies can be long and opaque.

Many genomic diagnostic services are not publicly subsidised. This privately funded model not only limits uptake, but also means that uptake is inequitable.<sup>5</sup>

The imperative to incentivise fixing this lottery is underpinned by the new NMP, which has as the first of its four pillars:

- "Equitable, timely, safe and reliable access to medicines and medicines-related services, at a cost that individuals and the community can afford."

### *System fragmentation remains*

One of the six reforms outlined in Schedule C of the NHRA is "nationally cohesive health technology assessment."<sup>6</sup> Similarly, the *NHRA Long Term Health Reforms Roadmap* acknowledges:

- "The current approach to HTA to inform policy investment and disinvestment decisions in Australia is fragmented. It does not support coordinated and timely responses to rapidly changing, emerging, and disruptive technologies, including high-cost and highly specialised therapies and services.

Separate HTA processes exist across all levels of the health system, and across levels of government. This duplicates effort, creates inefficiencies and inconsistent advice, and delays access to innovative and emerging technologies."<sup>7</sup>

Despite this recognition, and the separate HTA Review underway, system fragmentation remains. It cannot be solved by the HTA Review alone.

The high cost of many of these treatments often leads to negotiations between pharmaceutical companies and reimbursement bodies to determine the reimbursement amount. In some cases, the

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<sup>4</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.xvii.

<sup>5</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.84.

<sup>6</sup> *National Health Reform Agreement – Addendum 2020-25*, p.56:

[https://federalfinancialrelations.gov.au/sites/federalfinancialrelations.gov.au/files/2021-07/NHRA\\_2020-25\\_Addendum\\_consolidated.pdf](https://federalfinancialrelations.gov.au/sites/federalfinancialrelations.gov.au/files/2021-07/NHRA_2020-25_Addendum_consolidated.pdf)

<sup>7</sup> *National Health Reform Agreement (NHRA) – Long-term Health Reforms: Roadmap*,

<https://www.health.gov.au/sites/default/files/documents/2021/10/national-health-reform-agreement-nhra-long-term-health-reforms-roadmap.pdf>, p.7.

costs are shifted to other areas of the healthcare system, such as hospitals or insurance plans, to accommodate the high price of CAR T-cell therapies.

For example, consultations with stakeholders across the sector during the development of *State of the Nation 2023* found that the establishment of CAR T-cell centres has been ad hoc, complicated by funding models, and affected by cost-shifting and competition.<sup>8</sup>

CAR T-cell therapies are reliant on a complicated funding mix from both the Federal and state and territory governments. Eight centres provide CAR T-cell therapy in Australia, but are concentrated in NSW (4 centres), Victoria (2 centres) and Queensland (2 centres), with an unclear process for establishing sites.<sup>9</sup> To date, funding for these services has been based on the National Health Reform Agreement (50% state and 50% Federal).

### Access is impacted

Partially as a result of some of these challenges, many potentially life-saving therapies available overseas are not available in Australia.

Appendix A identifies many blood cancer treatments and diagnostics that are used overseas but not in routine clinical practice in Australia.

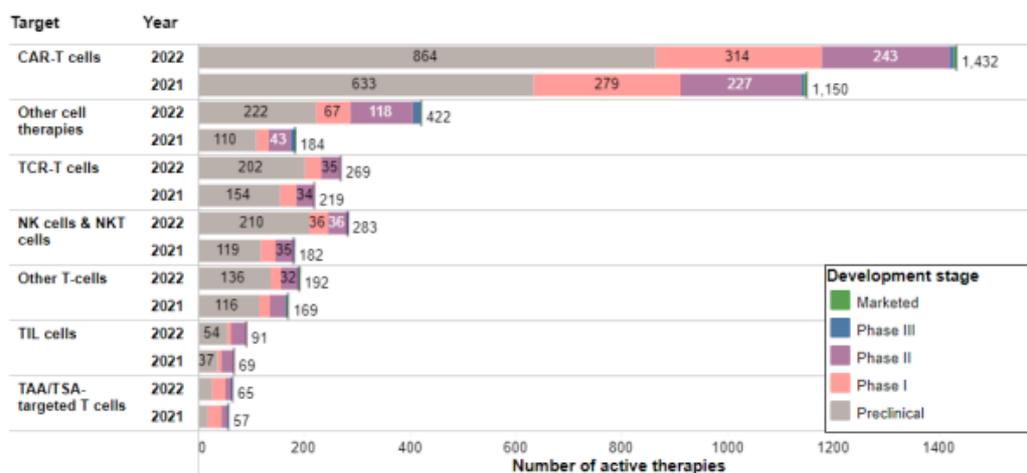
While the specific reasons for the delay vary from therapy to therapy, they generally are some combination of:

- Lack of evidence to support listing / repurposing
- Lack of incentives for companies to seek listing
- Time, cost, skills and IP/data barriers to clinician-led listings
- Barriers arising from high cost of therapies
- Complexity arising from federated model of health care.

### Preparing for the future

There is a significant pipeline of cell and gene therapies being developed globally, ultimately attempting to enter mainstream clinical practice.

Figure 2: A wave of CAR T-cell therapies on the horizon<sup>10</sup>



<sup>8</sup> State of the Nation, p.78 – [https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation\\_Final-Report\\_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf](https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation_Final-Report_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf)

<sup>9</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.78.

<sup>10</sup> [https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation\\_Final-Report\\_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf](https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation_Final-Report_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf), p.83.

Australia's future healthcare regulatory and reimbursement eco-system must also cope with the speed at which we are becoming able to differentiate between different blood cancer types through genomics. This is underscored by a recent (March 2023) MSAC recommendation for NGS gene panel testing for genetic variants associated with blood cancers. Importantly, MSAC noted that 'genetic testing is now standard of care for patients with these types of malignancies.'

These newer therapies are compounding existing access gaps due to their high cost.

Further, improving access to therapies will also necessitate ensuring we maintain a skilled and well-resourced workforce. This reflects one of the four central pillars of the new NMP: "Collaborative, innovative and sustainable medicines industry and research sectors with the capability, capacity and expertise to respond to current and future health needs."<sup>11</sup>

This workforce need is particularly important for newer therapies. Consultation undertaken as part of *State of the Nation 2023* showed that clinician stakeholders feel today's workforce is not adequately resourced to deliver novel medicines and CAR T-cell therapies.<sup>12</sup>

### *Towards a national policy*

Australia needs a national policy for the commissioning, funding and governance of CAR T-cell and other cellular and genetic therapies.

These therapies could be beneficial for many patients, but collaboration among the Federal and State and Territory Governments is required to develop a policy that:

- ensures a coordinated national approach to supporting the development of cellular and genetic therapies
- overcomes challenges caused by current funding arrangements, e.g. as seen with CAR T-cell therapies.
- draws from international initiatives, such as the FDA's Regenerative Medicine Advanced Therapy Designation.

*"When it comes down to the wire for patients, the lengthy approval/denial/repeat process in Australia is devastating. Especially when new drugs and procedures have been approved and are successfully in use overseas (usually the US and Europe), such as, lately, CAR-T and bi-specific antibodies.*

Blood cancer patient – Leukaemia Foundation Consumer Engagement Group

*'Disparity between states on availability of CAR T [has been an issue], also cut-off ages for treatments varies between states.'*

Blood cancer patient – State of the Nation 2023

### **RECOMMENDATION**

- That the NHRA Review recommends Australia develops a national policy for the commissioning, funding and governance of CAR T-cell and other cellular and genetic therapies.

<sup>11</sup> *National Medicines Policy*, <https://www.health.gov.au/sites/default/files/2022-12/national-medicines-policy.pdf>

<sup>12</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.53.

### 3. Elevate Patient Reported Outcomes

Patient Reported Outcomes (PROs) are not adequately embedded in our healthcare system, including the NHRA and associated data gathering, measurement and reporting.

The NHRA Roadmap commits governments to striving to "promote the use of Patient Reported Measures to understand what patients value and improve patient experiences and outcomes."<sup>13</sup>

However, PROs need to be central to data collection and embedded more strongly into the next NHRA. This should be mandated and codified.

Use of PROs allows the lived experience of patients to be better reflected in healthcare decisions. Specifically, they can:<sup>14</sup>

- Improve effectiveness and efficiency of patient and clinician engagement
- Monitor, evaluate, benchmark and improve the development of support services
- Inform government investment decisions.

In the context of cancers, PROs support engagement during what can be an intense period of diagnosis and treatment. Furthermore, progress in the type and number of therapies available to treat cancer mean that some cancers are becoming long-term chronic conditions, instead of acute, life-threatening ones. This means treatment is required for longer, and the experiences of patients needs to be captured across a longer period.

Recognising the importance and value of PROs, the *National Strategic Action Plan for Blood Cancers* called for a national system for patient reported outcomes in blood cancer. This been adopted in the draft *Australian Cancer Plan*, which includes the following action (4.2.1):

- "Design and embed patient reported experience and PROs into national performance monitoring and reporting for all providers, to assess services for all population groups and establish an evidence base."<sup>15</sup>

#### *Case study: Incorporating consumer needs – The United Kingdom*

The National Institute for Health and Care Excellence (NICE) in the UK has made revisions to its HTA processes to better capture consumer views in HTA processes.

The reforms are designed to allow greater flexibility over decisions about value for money and consideration of a broader evidence base.<sup>16</sup>

NICE's reforms include<sup>17</sup>:

- *Adopting new approaches to the evidence* considered by NICE in HTA. This includes greater consideration of real-world evidence from the lived experiences of patients.

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<sup>13</sup> <https://www.health.gov.au/sites/default/files/documents/2021/10/national-health-reform-agreement-nhra-long-term-health-reforms-roadmap.pdf>, p.18.

<sup>14</sup> [https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation\\_Final-Report\\_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf](https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation_Final-Report_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf)

<sup>15</sup> <https://engage.australiancancerplan.gov.au/projects/download/12702/ProjectDocument>

<sup>16</sup> <https://www.nice.org.uk/news/article/nice-signals-commitment-to-greater-flexibility-in-its-evaluation-of-promising-new-health-technologies-and-making-patient-access-fairer>

<sup>17</sup> *Changes we're making to health technology evaluation*, NICE: <https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation>

NICE's new "Real-world evidence framework" (June 2022) accepts that "real-world data can improve our understanding of health and social care delivery, patient health and experiences, and the effects of interventions on patient and system outcomes in routine settings."

The Framework acknowledges NICE wants to "use real-world data to resolve gaps in knowledge and drive forward access to innovations for patients."<sup>18</sup>

The Framework, which could be drawn on by Australian HTA authorities, drives better access by:

- "identifying when real-world data can be used to reduce uncertainties and improve guidance
- clearly describing best-practices for planning, conducting and reporting real-world evidence studies to improve the quality and transparency of evidence."<sup>19</sup>
- Allowing more flexibility for NICE's independent committees in cases where it is particularly difficult to generate enough evidence.

In acknowledgment of the challenges with generating evidence for some treatments – particularly those regarding children, rare diseases and innovative treatments – NICE's committees will now be able to better manage and consider uncertainty.

For example, the new *Health Technology Evaluations: The Manual* states:

- "In these [children, rare diseases and innovative treatments] specific circumstances, the committee may be able to make recommendations accepting a higher degree of uncertainty. The committee will consider how the nature of the condition or technology(s) affects the ability to generate high-quality evidence before applying greater flexibility."<sup>20</sup>
- Giving additional weight to health benefits in the most severe conditions, to allow more equitable access to treatments for these conditions (not just end-of-life).

## RECOMMENDATION

- That the NHRA Review recommends development of a national system for Patient Reported Outcomes, which should be aligned to the PRO recommendations in the Australian Cancer Plan (when publicly available).

<sup>18</sup> NICE "Real-world evidence framework," <https://www.nice.org.uk/corporate/ecd9/chapter/overview>

<sup>19</sup> NICE "Real-world evidence framework," <https://www.nice.org.uk/corporate/ecd9/chapter/overview>

<sup>20</sup> *NICE Manual 2022*, <https://www.nice.org.uk/process/pmg36/resources/nice-health-technology-evaluations-the-manual-pdf-72286779244741>

#### 4. Stem cell transplant and donation funding arrangements

Stem cell transplantation is one example where health reform agreements have not fixed an underlying patient access issue.

##### *The need for reform*

Stem cell (bone marrow) transplants are relatively unique to blood cancer. Despite being potentially curative, stem cell transplants are associated with serious mortality and morbidity risks. Patients receiving transplants are required to spend substantial time in hospital and stay nearby post-transplant.<sup>21</sup>

For many blood cancer patients, a stem cell transplant is the last resort therapy which, if successful, provides a long-term cure for their blood cancer. A patient must be matched with an appropriate Haemopoietic Progenitor Cell (HPC) donor, just as a blood transfusion can only be received from a person with a matching blood type.<sup>22</sup>

Every year, more than 600 Australians with blood cancer will need donated stem cells for a potentially life-saving allogeneic transplant. Some patients find a matched donor in their family, but more than half will need stem cells from a matched but unrelated individual.

However, not enough suitable Australians are registered as a donor, and this has been declining over time. As a result, about three in four of all stem cell donations are sourced from overseas donors.

Relying on overseas donations also means patients from various ethnic backgrounds are not well represented on international registries, like First Nations people and Pacific Islanders, and find it more difficult to find a suitably matched donor.

##### *A national, coordinated funding effort is required*

There is considerable room to better optimise current stem cell donor processes and funding, both at the State and territory level, and nationally.

A government-commissioned PwC review found Governance of the HPC sector is fragmented, with responsibilities spread across many different organisations.

Highlighting the issues for diverse populations, the National Strategic Action Plan for Blood Cancers, endorsed by the Federal and all State and Territory Governments in 2020, identifies the need to improve access for Indigenous and CALD people (Actions 1.6 and 1.7) to the stem cell register as part of actions required to support high-risk groups.<sup>23</sup>

Similarly, one of the six Strategic Objectives in the draft new Australian Cancer Plan is "Achieving Equity in Cancer Outcomes for Aboriginal and Torres Strait Islander People."<sup>24</sup>

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<sup>21</sup> *State of the Nation: Blood Cancers in Australia Report 2023*: [https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation\\_Final-Report\\_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf](https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation_Final-Report_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf)

<sup>22</sup> *State of the Nation: Blood Cancers in Australia Report 2023*: [https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation\\_Final-Report\\_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf](https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation_Final-Report_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf)

<sup>23</sup> <https://www.leukaemia.org.au/national-action-plan/>

<sup>24</sup> <https://engage.australiancancerplan.gov.au/>

In February 2023, the Federal Health Minister underscored some of these challenges, and noted in Parliament that:

- "Bone marrow donations provide the stem cells that are required for stem cell transplants, life-saving treatments today for people who are fighting leukaemia and a range of other blood cancers...

Part of the problem is that no single government between the Commonwealth and the states and territories has sole responsibility or sole authority to make sure that Australia keeps pace with the rest of the world and with these advances in technology."<sup>25</sup>

At the Health Ministers' Meeting on 24 February 2023, the Federal Health Minister and State and Territory Health Ministers agreed that:

- "Bone Marrow – Health Ministers approved immediate release of \$1 million from the Cord Blood Export Revenue fund to increase bone marrow donation recruitment, through both blood testing and cheek swabbing. Proposals for scaling bone marrow donation recruitment nationally are currently being considered. Health Ministers gave provisional approval for the release of further funds from the Cord Blood Export Revenue fund with details to be finalised following clinical advice, due in April 2023."<sup>26</sup>

As recognised by the Health Ministers' Meeting, increasing the stem cell donor pool requires a national effort. This is in part due to the challenges and probabilities in finding a match, and because of the complex funding and delivery arrangements across the country.

#### **Recommendation**

- That as part of the NHRA Review, the Reviewers consider current funding arrangements for stem cell donations and transplants and identify opportunities to harmonise these across all jurisdictions.

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<sup>25</sup> Hansard, 7 February 2023,

[https://www.aph.gov.au/Parliamentary\\_Business/Hansard/Hansard\\_Display?bid=chamber/hansardr/26418/&sid=0080](https://www.aph.gov.au/Parliamentary_Business/Hansard/Hansard_Display?bid=chamber/hansardr/26418/&sid=0080)

<sup>26</sup> <https://www.health.gov.au/sites/default/files/2023-02/health-ministers-meeting-communique-24-february-2023.pdf>

## APPENDIX A: Access gaps in blood cancer diagnosis and treatment

Sub-type	Diagnosis or monitoring testing	First line treatment options	Relapsed and refractory treatment options
<b>ALL</b>	<p>Minimum Residual Disease testing and monitoring which allows for assessment of the depth of remission following therapy and optimise the use of allogeneic stem cell transplantation.</p> <p>Lack of funding options for advanced diagnostics (PCR, genomic array, NGS). As a result, several WHO defined ALL sub-entities are not routinely discoverable with current standard of care diagnostics, precluding selection of optimal therapy</p>	<p>blinatumomab                      imatinib for Ph-like ALL                      nilotinib for Ph-like ALL                      dasatinib for Ph-like ALL                      ponatinib for Ph-like ALL</p> <p>PBS restrictions on TKI use beyond 2 years of maintenance</p>	<p>CAR T-cell therapy for adults (over 25)</p> <p>Lack of options for relapsed T-ALL</p>
<b>AML</b>	<p>Lack of funding options for advanced diagnostics (MRD PCR and flow, microarray, NGS), which may impact on transplant decisions.</p>	<p>Many drugs are not funded for inpatient use despite being available on the PBS, which is restricted to outpatient medication use, with major examples including midostaurin, gemtuzumab, ozogomycin, venetoclax-azacitidine.</p> <p>gliteritinib (<i>FLT3</i> inhibitor)                      IDH-1 and 2 inhibitors (enasidenib, ivosfenib)</p>	<p>Certain MRD monitoring is not undertaken post diagnosis due to the lack of targeted treatment options for some of those markers if they were to relapse/progress.</p> <p>Cell therapy</p>
<b>Lower Risk MDS</b>	<p>Molecular testing unfunded/unavailable</p>	<p>Therapies that improve cytopaenias and /or transfusion dependence are largely unavailable or unfunded (e.g. EPO and luspatercept)</p>	<p>Therapies for relapsed / refractory MDS are nonfunded or unavailable</p>
<b>Intermediate and / or higher risk MDS</b>	<p>Molecular testing unfunded/unavailable and this may change risk and /or allotransplant decisions</p>	<p>Therapies for patients with 10-20% blasts are limited with CR rates &lt;50% and /or significant toxicities for combination therapy (e.g., venetoclax)</p>	<p>Nonfunded or unavailable for MDS phenotype unless patient progresses to AML</p>
<b>CML</b>	-	-	-

<b>CLL</b>	<p>Access to gene array and mutation screening by NGS (e.g. TP53)</p> <p>Frontline young patients no access to novel agents</p> <p>Inequitable access of genetic testing necessary to inform prognosis</p>	<p>acalabrutinib ± obinutuzumab, venetoclax + obinutuzumab for younger patients</p> <p>zanubrutinib</p> <p>ibrutinib</p>	<p>CAR-T cell therapy</p> <p>Triple refractory population- gaps in drug options</p> <p>Richter's Transformation - gaps in drug options</p> <p>Access to venetoclax retreatment</p> <p>Double refractory treatment options</p> <p>pirtobrutinib</p>
<b>Lymphoma</b>	Lack of funding options for advanced diagnostics (NGS)	critizotinib (ALK+Lymphoma,	azacitidine (T-Cell)

	and MRD) for lymphomas in general, including MYD88 status in WM, EZH2 status in Follicular lymphoma, P53 in CLL and MCL, Gene Expression Profiles in Diffuse Large B-Cell, (and T-cell) and Whole Exome Sequencing analysis of MYC, bcl 2 and bcl 6. FISH testing used to diagnose HGBL-double hit.	histiocytosis) for first line or relapse BRAF inhibitors for hairy cell leukaemia and histiocytosis. Thiotepa for transplant therapy in primary central nervous system lymphoma bortezomib for abc-DLBCL, and in mantle cell lymphoma polatuzumab for frontline DLBCL	CAR T-cell therapy (Follicular) CAR T-cell therapy (Richter's) CAR T-cell therapy (Mantle cell) Bi-specific antibodies including mosunetuzumab in FL, glofitimab and epcoritamab in DLBCL. tafasitamab and lenalidomide and polatuzumab BR for Diffuse Large B-Cell PD1 inhibitors for extranodal natural killer/T-cell EBV-specific CTL lines for extranodal natural killer/T-cell and other ebv-PTLDs pirtobrutinib in Mantle Cell Lymphoma tazemetostat in EZH2-mutated Follicular Lymphoma
<b>Hodgkin lymphoma</b>		brentuximab vedotin (CD30) in Advanced stage (AAVD)	
<b>Myeloma</b>	CT/PET and whole body STIR MRI skeletal surveys Lack of availability for MRI AND PET means that it is not possible to differentiate between smouldering myeloma and myeloma based on SLIM-CRAB criteria.  Lack of funding options for advanced diagnostics (NGS and MRD), including gene expression profile for baseline risk stratification.	bortezomib, lenalidomide, and dexamethasone (VRd) Bendamustine Daratumumab-RevDex for non-transplant eligible patients Specific regimens for high-risk myeloma, such as KCRD-daratumumab daratumumab-VTD induction for some patient cohorts	daratumumab-Pd KPd CAR-T cell therapy (both idecel and ciltacel) BCMA T cell engager - teclistamab

Source: State of the Nation, using data from NCCN, ESMO and NICE clinical guidelines; Department of Health, 2022, Application 1684, Genetic testing for variants associated with haematological malignancies; and National Action Plan for Blood Cancer, Chapters 3 and 5. <sup>27</sup>

<sup>27</sup> [https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation\\_Final-Report\\_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf](https://www.leukaemia.org.au/wp-content/uploads/2023/02/Leukaemia-Foundation_Final-Report_State-of-the-Nation-Blood-Cancers-in-Australia-Report-2023.pdf), pp75-77.