

March 2023

Science Strategy and Priorities Taskforce  
Department of Industry, Science and Resources  
10 Binara Street  
Canberra ACT 2601

Via email: [priorities@industry.gov.au](mailto:priorities@industry.gov.au)

Dear Science Strategy and Priorities Taskforce

**Re: Australia's National Science and Research Priorities and National Science Statement**

Thank you for the opportunity to provide a submission to the initial consultation on *Australia's National Science and Research Priorities and National Science Statement*.

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.

We welcome the impending update of *Australia's National Science and Research Priorities and National Science Statement*.

Australia has developed a much clearer understanding of the social, emotional and economic impacts from blood cancers since publication of the Priorities in 2015 – and of the need to take action.

This submission draws on the wealth of evidence pointing to how best to leverage medical science to address these impacts and challenges. That evidence base includes but is not limited to:

- Australia's first ever *National Strategic Action Plan for Blood Cancer* (2020), and
- the recent *State of the Nation: Blood Cancers in Australia Report 2023* (February 2023).

Unlike other cancers and diseases, blood cancers cannot be prevented or screened for.

Medical research and scientific discovery, and the translation of this into accessible treatments for patients, therefore has a vital role to play in saving and improving patients' lives.

The consultation documentation indicates the Priorities and Statement will 'provide direction for our science system' and 'reflect Australia's ambitions and challenges.' Improving the health of our population should be both a direction and ambition for the Australian Government.

Australia is well-placed to leverage recent rapid scientific advances and its pre-existing research expertise to cement our place as a world leader in medical science. In doing so, we have the chance to improve and save the lives of those living with blood and other cancers.

## 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.

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. Establishing genomics as the standard of care

### The opportunity

Rapid advances currently occurring in genomics mean we can potentially revolutionise health care.

Genomic profiling enables more accurate subtype diagnosis. This allows clinicians to improve survival outcomes by more precisely matching patients to targeted therapies.<sup>2</sup>

Accurate diagnosis is especially critical for blood cancers.

Cytogenetic and molecular genomic testing are increasingly being used alongside traditional tests because they provide a more accurate diagnosis by sub-type.

Cutting edge genomics such as next generation sequencing (NGS) and minimal residual disease (MRD) testing could magnify these benefits through capturing large amounts of genomic

---

<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.

<sup>2</sup> National Strategic Action Plan for Blood Cancers, 2020, [https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer\\_June-2020.pdf](https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer_June-2020.pdf)

information and deploying highly sensitive testing methods.<sup>3</sup> MSAC recently (March 2023) supported this testing being Medicare-funded, noting a 'modest financial impact' to the MBS. Tellingly, the summary document notes:

- **“MSAC supported public funding of MRD testing because it is the established standard of care in these patients, and to correct the current inequity of access to the testing required to access PBS-listed blinatumomab”** (because patients are currently paying out-of-pocket or seeking public hospital funding via the PBS)<sup>4</sup> – *MSAC, March 2023*

Examples of where this is being applied are the OMICO ProSPeCT program<sup>5</sup> and the ZERO childhood cancer project.<sup>6</sup>

The potential impact of harnessing this technology is demonstrable and profound. Our recent 'State of the Nation' report contains new data showing 31 per cent of blood cancer patients who had a genomic test had their diagnosis and treatment plan altered.<sup>7</sup>

Further, Appendix A recounts how a patient who was given three weeks to live has recently had his life turned around in a current (March 2023) genomics trial funded by the Leukaemia Foundation and Tour de Cure, thanks to genomics profiling identifying a more targeted therapy.

The Zimmerman Inquiry agreed with this potential, finding that:

- "...affordable access to genomic testing is needed **not only for patients but for the future of Australia's health system**. Therefore the Australian Government should establish a National Genomics Testing Program to provide equitable access to genomics testing nationwide, including provision for genomics counselling for all patients."<sup>8</sup>

Similarly, in a separate, recent MSAC recommendation, MSAC provided landmark support 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'
- 'the [5th edition of the World Health Organization \(WHO\) Classification of Haematolymphoid Tumours: Lymphoid Neoplasms \(2022\)](#) recommends molecular testing for establishing a comprehensive diagnosis'
- 'without genetic testing, patients may be incorrectly diagnosed and potentially receive ineffective or incorrect treatment'<sup>9</sup> – *MSAC, March 2023*

Developing our genomics capability in turn provides a powerful tool to drive further research.

---

<sup>3</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>4</sup> MSAC public summary document: *Application No. 1707 – clonoSEQ® and mpFC for the detection of measurable residual disease (MRD) in acute lymphoblastic leukaemia (ALL)*;

[http://www.msac.gov.au/internet/msac/publishing.nsf/Content/67D026849586C408CA25879B008371EC/\\$File/1707%20Final%20PSD-Nov2022\\_redacted.pdf](http://www.msac.gov.au/internet/msac/publishing.nsf/Content/67D026849586C408CA25879B008371EC/$File/1707%20Final%20PSD-Nov2022_redacted.pdf)

<sup>5</sup> <https://www.omico.com.au/prospect/>

<sup>6</sup> <https://www.zerochildhoodcancer.org.au/>

<sup>7</sup> *State of the Nation: Blood Cancers in Australia Report 2023*

<sup>8</sup> New Frontiers report, p.229: [https://parlinfo.aph.gov.au/parlInfo/download/committees/reportrep/024755/toc\\_pdf/TheNewFrontier-DeliveringbetterhealthforallAustralians.pdf;fileType=application%2Fpdf](https://parlinfo.aph.gov.au/parlInfo/download/committees/reportrep/024755/toc_pdf/TheNewFrontier-DeliveringbetterhealthforallAustralians.pdf;fileType=application%2Fpdf)

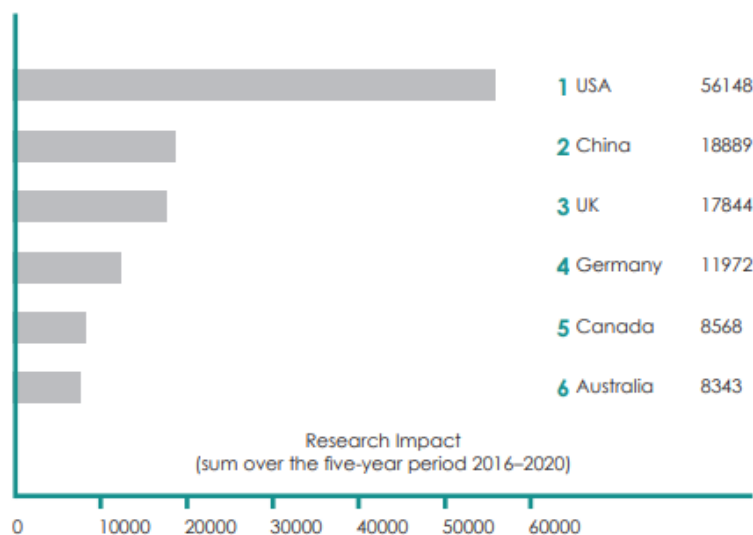
<sup>9</sup> Medical Services Advisory Committee (MSAC) Public Summary Document: *Application No. 1684 – Genetic testing for variants associated with haematological malignancies*

[http://www.msac.gov.au/internet/msac/publishing.nsf/Content/0E3364FCF94B9002CA25874F00283CE5/\\$File/1684%20Final%20PSD-Nov%202022.pdf](http://www.msac.gov.au/internet/msac/publishing.nsf/Content/0E3364FCF94B9002CA25874F00283CE5/$File/1684%20Final%20PSD-Nov%202022.pdf)

The Department of Industry, Science and Resources (DISR) has already recently acknowledged genomics' significance, by identifying it as a 'critical technology' in the draft *List of Critical Technologies in the National Interest (2022)*.<sup>10</sup>

As DISR states, Australia is also well-placed to contribute to this field. Australia has the sixth highest research impact globally, and it is an area that Australia is particularly strong in.<sup>11</sup>

Figure 1: Research impact for genomics (volume and level of interest in publications)<sup>12</sup>



### Challenges

Given the benefits, genomic testing should be the standard of blood cancer care – but this is not happening.

Instead, the option to access to genomic diagnostics in Australia is limited,<sup>13</sup> their usage is inconsistent across the population, and ultimately few patients are undergoing genomic testing.<sup>14</sup>

A disconnect is occurring between genomics delivery and patient care. The *State of the Nation* report shows:

- only one in five patients (21%) knew that a genomic test was used to confirm their diagnosis
- 43% did not know if a genomic test was used to confirm their diagnosis.

Many genomic diagnostic services are not publicly subsidised. This privately funded model not only limits uptake, but also means that uptake is inequitable.<sup>15</sup>

Reporting times remain a key issue. The reporting time range for FISH (fluorescence in situ hybridisation) has been previously calculated at one to 56 calendar days, although FISH testing for myeloma has recently been reported as regularly taking up to six months.<sup>16</sup>

<sup>10</sup> *List of Critical Technologies in the National Interest, 2022*: [https://storage.googleapis.com/converlens-au-industry/industry/p/prj213f6fb4eef13398228aa/public\\_assets/critical-technologies-2022-consultation-paper.pdf](https://storage.googleapis.com/converlens-au-industry/industry/p/prj213f6fb4eef13398228aa/public_assets/critical-technologies-2022-consultation-paper.pdf)

<sup>11</sup> *Critical Technology Profiles, 2022*: [https://storage.googleapis.com/converlens-au-industry/industry/p/prj213f6fb4eef13398228aa/public\\_assets/Critical-Technology-Profiles.pdf](https://storage.googleapis.com/converlens-au-industry/industry/p/prj213f6fb4eef13398228aa/public_assets/Critical-Technology-Profiles.pdf)

<sup>12</sup> [https://storage.googleapis.com/converlens-au-industry/industry/p/prj213f6fb4eef13398228aa/public\\_assets/Critical-Technology-Profiles.pdf](https://storage.googleapis.com/converlens-au-industry/industry/p/prj213f6fb4eef13398228aa/public_assets/Critical-Technology-Profiles.pdf)  
<sup>13</sup> National Strategic Action Plan for Blood Cancers, 2020, p.35: [https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer\\_June-2020.pdf](https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer_June-2020.pdf)

<sup>14</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.85.

<sup>15</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.84.

<sup>16</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.50.

The limited use is slowing the development of data to inform research efforts.<sup>17</sup>

### Recommendations

Genomics, and Australia's relative strength in this area of science, should be recognised as an opportunity to improve health outcomes and identified as a scientific and research priority.

As identified in the *National Strategic Action Plan for Blood Cancers*, genomic testing (precision medicine) should be embedded as the standard of care.<sup>18</sup> Inclusion of genomics in the revitalised Priorities and Statement will help support investment, which will ultimately support and help realise improvements in clinical practice and blood cancer research efforts.

This can help inform further initiatives that could support the development of our genomics knowledge base and achieve greater genomic testing reach.

Adopting current precedent already established by MSAC could speed up availability of genomic and genetic testing to blood cancer patients. At its August 2019 meeting, MSAC noted specific WHO Guidelines and International Myeloma Working Group consensus on risk stratification in multiple myeloma provide the appropriate standards of clinical care for Australian patients.<sup>19</sup>

Australia can capitalize on recent advances and expert advice (e.g. MSAC's March 2023 recommendations) that would help advance genomic testing in Australia.

*"Genomic testing is critical. There's no comprehensive coordinated testing process, which needs to be addressed as it underpins everything we do. An accurate diagnosis is absolutely necessary for provision of best quality care."* – 'State of the Nation' respondent

*"The complexity of blood cancers needs to be clearly understood. We know they're complex... This really speaks to the need for precision medicine and genomic testing."* – 'State of the Nation' respondent

#### **RECOMMENDATIONS**

1. As part of the summary developed for this current consultation, genomic science and research should be recognised as a key opportunity to greatly improve health (and other) outcomes.
2. The final Priorities should help facilitate genomics becoming the standard of care through encouraging initiatives that support Australia's scientists undertake genomics study, and facilitating the availability of genomics testing to scientists, clinicians and patients across the country.
3. The Priorities should support and include reference to a new Centre for Precision Medicines and Rare Diseases to help drive Australian genomics.

<sup>17</sup> National Strategic Action Plan for Blood Cancers, 2020, p.35: [https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer\\_June-2020.pdf](https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer_June-2020.pdf)

<sup>18</sup> National Strategic Action Plan for Blood Cancers, 2020, p.35: [https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer\\_June-2020.pdf](https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer_June-2020.pdf)

<sup>19</sup> State of the Nation: Blood Cancers in Australia Report 2023, p.50.

## 2. Accelerating medical research and discovery

### The opportunity

Investing in medical research can help save and improve lives.

As identified in the *National Strategic Action Plan for Blood Cancers*, new investment in genomics, microbiota, diagnostics, immunotherapies, targeted therapies and cellular therapies can potentially treat blood cancer subtypes that currently have no cure and poor five-year survival rates.<sup>20</sup>

Research breakthroughs have already led to significant improvements in blood cancer mortality rates. In recent decades, cures have been discovered for some paediatric blood cancers, and a new treatment (tyrosine kinase inhibitor therapies) has significantly improved life expectancies for chronic myeloid leukaemia patients.<sup>21</sup>

Similarly, the Victorian Cancer Registry has recently found that "improvement in 5-year survival for blood cancers is in large part due to discovery of novel targeted therapies."<sup>22</sup>

Australian researchers have demonstrated their ability to undertake high quality research, such as through the development of the breakthrough anti-blood cancer drug Venetoclax.

Supporting medical research also delivers broader economic benefits. KPMG have shown that every \$1 spent on our medical research sector returns \$3.90 in health benefits.<sup>23</sup>

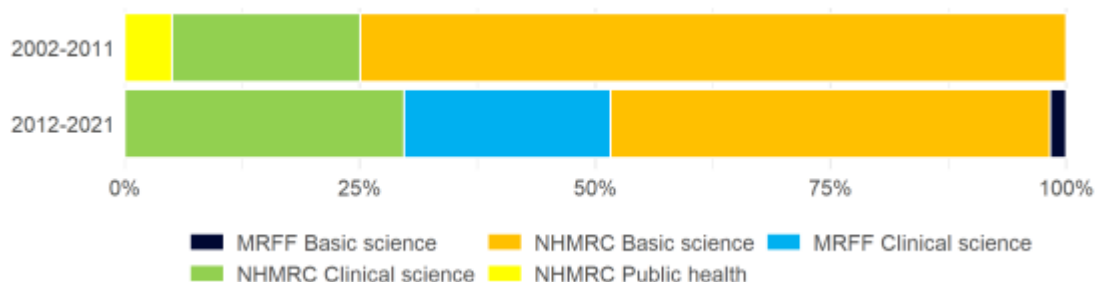
Separately, reviews have shown the potential for novel health technologies (e.g. cell and gene therapies) to lead to more cost effective treatments in the future given they can be used to treat multiple diseases such as genetic diseases, cancers and infectious diseases.<sup>24</sup>

### Challenges and barriers

Blood cancer research is relatively underfunded, with blood cancers such as lymphoma and myeloma receiving relatively low funding support.<sup>25</sup>

Further, government funding for basic research has declined from 75 per cent during 2002-2011 to only 48 per cent during 2012-2021.<sup>26</sup>

Figure 2: NHMRC and MRFF funding for clinical and basic sciences, blood cancers



<sup>20</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.42.

<sup>21</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.50.

<sup>22</sup> Victorian Cancer Registry, 2022, *Cancer in Victoria 2021*.

<sup>23</sup> KPMG, *Economic impact of medical research*, 2018, p.6.

<sup>24</sup> New Frontiers report, p.229: [https://parlinfo.aph.gov.au/parlInfo/download/committees/reportrep/024755/toc\\_pdf/TheNewFrontier-DeliveringbetterhealthforallAustralians.pdf;fileType=application%2Fpdf](https://parlinfo.aph.gov.au/parlInfo/download/committees/reportrep/024755/toc_pdf/TheNewFrontier-DeliveringbetterhealthforallAustralians.pdf;fileType=application%2Fpdf)

<sup>25</sup> <https://www.canceraustralia.gov.au/publications-and-resources/cancer-australia-publications/cancer-research-australia-overview-funding-cancer-research-projects-and-programs-australia-2012-2020>

<sup>26</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.65.

More broadly, Australia's R&D sector funding (1.8% of GDP) is well below the OECD average (2.67%).<sup>27</sup>

This limited funding reduces the potential impact of our medical research sector. It also risks a subsequent reduction in our scientific workforce.

To fulfil the promise of our medical research potential, we require a skilled and well-resourced workforce. This reflects one of the four central pillars of the new *National Medicines Policy* (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>28</sup>

Yet the State of the Nation report found:

- "Clinicians and researchers indicated that limited funding for research is contributing to an **existential issue for Australian researchers**, particularly in basic science and early phase research."<sup>29</sup>
- Attrition of the clinical trials workforce is **impeding research activity**.<sup>30</sup>
- "Clinicians believe that **today's workforce is inadequately resourced to deliver novel medicines** and CAR T-cell therapies, meaning Australia's propensity to adopt this technology in a sustainable manner is presently limited."<sup>31</sup>

Last year, only 11% of the NHMRC's Ideas Grant applications were funded. Only 15% went to early- and mid-career researchers.<sup>32</sup>

Meanwhile, the US National Institutes of Health, EU Horizons and China's Thousand Talents Program are significantly increasing their research budgets and becoming even more attractive for researchers.<sup>33</sup>

These challenges are compounded by a pre-existing STEM (science, technology, engineering and mathematics) skills and teacher shortage.<sup>34</sup> For example, the Australian Academy of Technological Sciences and Engineering has found the 'status of mathematics education is an issue of national concern,' with around one fifth of advertised secondary mathematics teaching vacancies in Victoria going unfilled.<sup>35</sup>

The establishment of Jobs and Skills Australia is welcome, but the challenge of STEM shortages for the future prosecution of Australia's science and research agenda remains.

Finally, the *Australian Medical Research and Innovation Priorities 2022-2024* identify the need for 'facilitating collaborations between the research sector, industry and community', given Australia's world-class research output is not reflected in levels of research translation and commercialisation.<sup>36</sup>

The national and international collaborations over several decades that led to Venetoclax being available to patients underscores the importance of partnerships and supporting research

---

<sup>27</sup> ATSE Pre-Budget Submission, p.14: <https://www.atse.org.au/wp-content/uploads/2023/01/ATSE-SUB-230127-Pre-budget-submission.pdf>

<sup>28</sup> <https://www.health.gov.au/sites/default/files/2022-12/national-medicines-policy.pdf>

<sup>29</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.66.

<sup>30</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.66.

<sup>31</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.53.

<sup>32</sup> Budget Proposal 2023-24, [https://www.aamri.org.au/wp-content/uploads/2023/02/AAMRI\\_Budget\\_Proposal\\_2023.pdf](https://www.aamri.org.au/wp-content/uploads/2023/02/AAMRI_Budget_Proposal_2023.pdf)

<sup>33</sup> [https://www.aamri.org.au/wp-content/uploads/2023/02/AAMRI\\_Budget\\_Proposal\\_2023.pdf](https://www.aamri.org.au/wp-content/uploads/2023/02/AAMRI_Budget_Proposal_2023.pdf)

<sup>34</sup> <https://www.atse.org.au/wp-content/uploads/2022/08/SBM-2022-08-08-Jobs-and-Skills-Australia-bill-v3.pdf>

<sup>35</sup> p.13: <https://www.atse.org.au/wp-content/uploads/2022/10/ATSE-Education-Report-for-web.pdf>

<sup>36</sup> *Australian Medical Research and Innovation Priorities*, <https://www.health.gov.au/sites/default/files/documents/2022/11/australian-medical-research-and-innovation-priorities-2022-2024.pdf>

translation. NHMRC's welcome and first *NHMRC Research Translation Strategy 2022–2025* was recently published, and its first two priorities underscore this focus:

- Encourage partnerships between researchers and end-users
- Encourage and build capacity and capability in research translation.<sup>37</sup>

### Recommendations

Australia has an opportunity to harness its expertise and drive further breakthroughs in medical research and novel therapeutics. Investment in STEM disciplines and researcher capacity is critical to capitalise on this expertise and create an ecosystem that is capable of taking on the research challenges of tomorrow from fundamental science through to translational and clinical practice.

The Leukaemia Foundation is funding the development of a Research Roadmap, to map out a 10-year plan to develop an ecosystem that accelerates breakthrough blood cancer research in Australia. The aim is for this roadmap to inform state and federal governments and the broader blood cancer community.

#### **RECOMMENDATION**

4. The Science and Research Priorities and Science Statement should reflect the importance of continued investment in medical research – including basic, clinical and translational – to Australia's continued contributions to the discovery and development of life-saving novel therapeutics.
5. The Priorities and Statement should reflect the importance of prioritising and facilitating medical research in diseases with high impact (mortality, cost, intensity of treatment, etc.) such as blood cancers, and for funding allocations to also align with this.

### **3. Supporting access to new and emerging treatments and technologies**

#### The opportunity

Facilitating patient access to therapies and ensuring that access is equitable can help save and improve lives.

There have been some improvements in access arrangements for novel therapies in blood cancer. We now have an opportunity to build on this momentum.

This 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."

Moreover, the NMP subsequently commits the Commonwealth to "Deliver national health programs and regulatory functions that ensure equitable, timely, affordable, safe and reliable access to medicines and medicines-related services."<sup>38</sup>

<sup>37</sup> <https://www.nhmrc.gov.au/research-policy/research-translation-and-impact/research-translation-strategy-2022-2025>

<sup>38</sup> *National Medicines Policy*: <https://www.health.gov.au/sites/default/files/2022-12/national-medicines-policy.pdf>



The Health Technology Assessment Review currently underway is a further opportunity and lever to enable better access for patients.

### Challenges

Scientific advances are being made but Australians often wait to access and experience these.

Many life-saving blood cancer treatments, therapies and diagnostics aren't routinely used or available in Australia until many years after they are available overseas. Many of these are not subsidised despite being the best practice standard of care overseas, and in some instances even here in Australia. Developing the required evidence base in Australia for PBS/MBS reimbursement may be time-consuming, complex or expensive (or a combination of all three).<sup>39</sup>

As a result, Australia is still not in line with some of the international best practice when it comes to potentially life-saving cancer therapies.

Many of the newer diagnostics/treatments/therapies are available either only through 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.

There remains **inconsistent access to diagnostics and novel and specialised therapies** across Australia. That is, while some novel therapies are available, they are only available to some, 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>40</sup>

Our broader regulatory and reimbursement arrangements have to support the realisation of medical discoveries and thus the delivery of the benefits that flow through to the Australian population.

These access gaps are being magnified by new, high-cost cellular therapies such as CAR T-cell therapies, which are also 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>41</sup>

### Recommendations

We need to bring Australia into line with international best practice treatment by investing in research, and providing timely access to new treatments and therapies, as they're being discovered.

Improvements to Australia's regulatory processes, along with transparency on pharmaceutical companies' products and the complex system for medicines funding, would help do this.

---

<sup>39</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.77.

<sup>40</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.xvii.

<sup>41</sup> *State of the Nation: Blood Cancers in Australia Report 2023*, p.78.

Innovative thinking on access to new treatments and technologies is also needed and should be encouraged through the Priorities and Statement.

The Zimmerman Report supported previous Leukaemia Foundation recommendations for a 'Right to Trial Fund'. It recommended:

- "Federal Government...establish a fund to support patients, clinicians and non-profit organisations to sponsor registration and reimbursement applications where there is no realistic prospect of a company serving as sponsor, and where the Department of Health is otherwise supportive of the application (Recommendation 9)."

This commitment to best practice and providing timely access to treatments and therapies should extend to improving awareness of, and participation in, clinical trials as part of the development of a 'research-ready' culture in Australian clinical practice.

#### RECOMMENDATIONS

6. This consultation and the new Priorities and Statement should encourage investment in research that results in clinical practice change and provides Australian patients with access to the latest treatments and novel therapies, in particular cellular and immunotherapies.
7. The Priorities and Science Statement should acknowledge the opportunity Australia has to facilitate patient access to new therapies and ensure that this access is equitable.
8. The Priorities and Statement should encourage development of innovative new ways of encouraging further scientific discovery and ways of encouraging their uptake in Australia, such as the Right to Trial Fund and a national CAR-T policy.

Thank you for your consideration of the issues highlighted above. 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

## APPENDIX A: Case study – Real-world benefits of genomics for blood cancer patients

The Leukaemia Foundation and Tour de Cure are driving a genomics trial for people living with blood cancer through providing co-funding of \$1.8 million towards this program to pilot the screening of 240 patients.

The blood cancer genomics trial links genomic screening of patients who have failed or don't respond to treatment to multiple novel therapeutic combination treatments.

This initial investment has allowed screening and enrollment to open to patients at Royal Brisbane and Women's Hospital, and Royal Adelaide Hospital and SA Pathology providing molecular screening for the program across both states. The trial was awarded additional \$2.7 million in funding from the Medical Research Future Fund (MRFF) to expand to other sites.

Below is an account from the family member of a trial participant.

*"A huge thank you to LF [and Tour de Cure] for establishing the Blood Cancer Genomics trial as it has resulted in a life-saving treatment for my family member (parent with children).*

*The family member was diagnosed with T cell prolymphocytic leukaemia in early January (2023) and started standard treatment. Unfortunately, there was no response to the treatment and the condition worsened. It got to the point where even a 15 metres walk was a struggle. Just over a week ago, we were provided the news that our family member only had 3-4 weeks to live. The kids were taken out of school to spend what time they could with their parent.*

*Fortunately, at a meeting of haematologists immediately after the diagnosis, it was suggested an option was to go on the Blood Cancer Genomics trial as if frontline treatment failed.*

*The genomic screening provided critical information to commence treatment, and within a few days, the effect on the leukaemia has been nothing short of miraculous.*

*Within 5 days, lymphocyte count returned to the normal range, and within a week, our family member is better than they have been in months, even being able to ride an exercise bike for 15 minutes.*

*The effect this has had on our family and the kids is immeasurable, so many thanks for enabling this to happen.*