August 2023

Senate Community Affairs References Committee
PO Box 6100
Parliament House
Canberra ACT 2600

community.affairs.sen@aph.gov.au

Dear Senate Community Affairs References Committee

**Re: Inquiry into equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer**

Thank you for the opportunity to provide a submission to this Inquiry.

The Leukaemia Foundation is the only national organisation representing all Australians with blood cancer. We provide wraparound health services, fund leading-edge research and campaign for change alongside our community.

This submission draws on an evidence base that includes but is not limited to:

- our recent *State of the Nation: Blood Cancers in Australia Report 2023*, including a survey of over 4,600 Australians living with blood cancer, plus interviews with other stakeholders,
- Australia's first ever *National Strategic Action Plan for Blood Cancer* (2020),
- our Leukaemia Foundation Consumer Engagement Group,
- our ongoing daily interactions with, and support for, the patients receiving our services, including a July 2023 survey of over 300 patients using our services.

In line with the consultation documentation, this submission sequentially addresses:

1. barriers to screening and diagnosis,
2. barriers to accessing appropriate treatment;
3. the adequacy of support services after diagnosis
4. the adequacy of Commonwealth funding for research

Thank you for your consideration of the issues highlighted in this submission. We would be happy to discuss further and/or appear at subsequent public hearings, and can be contacted at

Sincerely,

**Chris Tanti**
Chief Executive Officer

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Leukaemia Foundation®

*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.

*About blood cancers*

- Blood cancers are a **complex group of individually rare diseases**, each with a host of genetically distinct subtypes requiring bespoke treatment and care.
- There are over 120 discrete blood cancers, including more than 40 unique sub-types of leukaemia. Other rare sub-types include myeloproliferative neoplasms (MPN) and myelodysplasia (MDS).
- 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.
- One in 3 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.
- 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.[1]



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## Executive Summary

Australia has a world-class health system but we can and should do better for people living with blood cancers.

There are over 120 discrete blood cancers, most of which are individually rare, non-preventable, non-screenable, difficult to diagnose, and difficult to treat.

Lives are being lost due to incomplete or non-existent formalised national treatment standards available to clinicians – especially for less common sub-types – resulting in nationally inconsistent survival outcomes. Some life-saving novel and specialised haematological therapies are either not available in Australia, or available only to those who can afford them.

Patients in non-metro areas or on lower incomes are disproportionately affected as their diagnostic, treatment and service options are often already limited.

This inequity is magnified by the underlying unique reality of treating blood cancers – they are already the most expensive cancers to treat, require long treatment periods, and are high-mortality.

Precision medicine is rapidly becoming a key component of cancer therapy but is being hindered by inadequate subsidisation, and structural challenges in our health system.

This journey is encapsulated by the recent experiences of Sarah, whose son passed 104 days after being diagnosed with a rare leukaemia (Appendix B). This included encountering:

- Logistical challenges being based on
- Difficulty finding fit-for-purpose services on the mainland
- Limited clinical knowledge of – and treatment options for – her son's rare leukaemia
- Difficulty finding support services
- Grappling with perceptions that childhood leukemia has a “cure”

Nonetheless, newer therapies, the ongoing evolution in subsidy arrangements, and standard-setting work underway means Australia has a significant opportunity to reduce this inequity and improve and save the lives of blood cancer patients. We can do this through:

1. Embedding genomics as the standard of care through greater public subsidy and investment
2. Supporting the implementation and ongoing development of optimal care pathways and clinical guidelines currently underway and establishing a national approach
3. Streamlining reimbursement pathways and establishing a ‘Right to Trial’ program to support systematic evidence development
4. Developing a national policy for the commissioning, funding and governance of CAR T-cell and other cellular and genetic therapies
5. Reforming financial support mechanisms, including patient assisted travel schemes
6. Leveraging disease-specific supportive care organisations such as the Leukaemia Foundation to fast-track the delivery of supportive care
7. Reflect the importance of prioritising and facilitating medical research in diseases with high impact (mortality, cost, intensity of treatment, etc.) such as blood cancers

The improvement in five-year survival for some blood cancers and the development by Australian researchers of Venetoclax demonstrate progress has been made. Now is the moment to invest further in treatments and research to reduce mortality and bridge outcome disparities – thus helping achieve consistent, high-quality care for all blood cancer patients regardless of their postcode.

## Summary of Recommendations

1. That the Committee remains cognisant that screening is not possible for blood (and other) cancers and that its final report notes the critical nature of alternative cancer control means to reduce blood cancer mortality.

2. Develop initiatives such as community engagement and education on signs and symptoms of blood cancer.

3. Develop symptom support tools for GPs aimed at improving prompt and accurate diagnosis and appropriate referral to specialists of low survival, non-screenable cancers, including blood cancers.

4. As identified in the National Strategic Action Plan for Blood Cancers (NAP action 2.2.1), review existing international diagnostics guidelines in blood cancer and develop Australian-specific guidelines for minimum and recommended testing requirements for different types of blood cancer in Australian healthcare settings. These guidelines should be integrated with clinical guidelines.

5. Ensure Australia keeps pace with international advances in the development of new diagnostic tools.

6. Genomic testing (precision medicine) should be embedded as the standard of care, through greater public subsidy, investment in diagnostic laboratories and their workforce, pathways for genetically trained pathologists and encouraging initiatives that support Australia's scientists undertake genomics study.

7. A national approach to the development and sustainable management of OCPs and clinical guidelines.

8. The creation of a 'Right to Trial' program to support systematic evidence development and provide a mechanism for the more systematic evaluation of off-label use and re-purposing of drugs.

9. That Government, subject to the outcomes of the HTA Review, identifies reimbursement pathways that allow for the use of earlier and more varied clinical data.

10. Through the Australian Cancer Plan or other instruments of government, enhance efforts to address the access challenges to new treatments and care for rare and low survival cancers.

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

12. The Inquiry considers the current funding arrangements for stem cell donations and transplants and identify opportunities to harmonise these across all jurisdictions.

13. Leverage the educational, navigation and supportive care resources of disease-specific supportive care organisations such as the Leukaemia Foundation to fast-track the delivery of supportive care.

14. Integrate community-led disease specific supportive care organisations such as the Leukaemia Foundation for blood cancer into the model of care.

15. Consult with all jurisdictions to reform PATS, including advocating for streamlined administrative processes, greater access to the schemes and PATS support for patients to participate in clinical trials (NAP recommendation 2.7.1).

16. Review options for the introduction of financial support mechanisms (such as a temporary disability payment) to support people with cancer and other serious illness who require temporary financial support (NAP recommendation 2.7.2).

17. Australia's research funding 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.

18. Define new investment in such areas as genomics, microbiota, diagnostics, immunotherapies, targeted therapies and cellular therapies which hold the potential to address the unmet needs of blood cancer subtypes for which there is currently no cure and where five-year survival is poor.

19. Develop a research strategy for low survival cancers, including emphasis on development of transformative therapies and a targeted support framework to execute this.

20. Through the implementation of the National Clinical Quality Registry Strategy, a review should be undertaken to evaluate options to improve the value and sustainability of blood cancer registries and Australian blood cancer patient data by extending the coverage of these registries. This should leverage from, and link to, existing administrative datasets and annotated samples stored at related biobanks, and enable linkages with international real world data endeavours.

21. Ensure alignment with the upcoming ACP, national strategy for health and medical research, NACCHO Cancer Plan and other initiatives, including identifying gaps applicable to blood cancers.

22. That the Committee recommends development of a national system for PROs, which should be aligned to the PRO recommendations in the ACP (when publicly available).

# 1 Barriers to screening and diagnosis

## 1.1 Barriers

### 1.1.1 Blood cancers cannot be screened for

Unlike many solid tumour cancers, blood cancers are neither preventable nor can they be easily identified through population screening.

Instead, reducing mortality relies on public health messaging to alert individuals to the signs and symptoms of blood cancer, access to prompt and accurate diagnosis, access to best practice treatment and care, and further scientific discovery that enhances treatment and care.

This underscores the importance of ensuring Australian blood cancer patients have access to effective diagnostics, treatment options and services.

The remainder of this section focuses on barriers to diagnosis.

RECOMMENDATION

1. That the Committee remains cognisant that screening is not possible for blood (and other) cancers and that its final report notes the critical nature of alternative cancer control means to reduce blood cancer mortality.

### 1.1.2 Blood cancers are difficult to diagnose

Blood cancers are uncommon, spontaneous, and challenging to readily diagnose in primary care settings. Many healthcare providers have limited experience with blood cancers, particularly the rarer subtypes.[2]

International studies show blood cancer diagnosis error rates can be high, and can occur more frequently in rare blood cancers.[3]

Similarly, new cancer registry data analysed in the *State of the Nation: Blood Cancer in Australia 2023* report commissioned by the Leukaemia Foundation, and the *National Strategic Action Plan for Blood Cancer* (NAP) developed by the Blood Cancer Taskforce, identified that:

- there is under-diagnosis and under-reporting of blood cancers in all Australian jurisdictions
- the incidence and prevalence of blood cancers in Aboriginal and Torres Strait Islander communities is likely significantly under-diagnosed and under-reported.

These diagnostic challenges are confirmed in patients' lived experiences. In the survey of over 4,600 Australian blood cancer patients conducted as part of *State of the Nation 2023*, improving diagnostic capability was patients' highest reform priority within the 'health services reform and access to best practice care' category.[4] Patients noted that:

> *"Pre-diagnosis, I was sent to numerous specialists who conducted many tests which were out of pocket. This went on for six years until I was finally diagnosed...I became very emotionally damaged during all of this as I wasn't believed even though I had all the symptoms of MDS and then some!"*[5]
> – Blood Cancer Patient

> *"There seems to be a poor recognition of blood cancer symptoms in general practice...I presented to [my GP] for four months with symptoms that were dismissed as asthma and eczema. I think this is the biggest issue facing blood cancer patients."*[6]

– Blood Cancer Patient

*" I should have trusted my intuition...Having a sick child – dealing with the healthcare system – is about learning to speak up to the experts."*[7] – Mother of Blood Cancer Patient

*"My cancer was dismissed by two separate senior GPs. The third GP I saw (a junior Dr) was the first one to do a physical examination and noticed my enlarged lymph nodes immediately. Without his thorough care who knows how long my diagnosis would have been delayed."*[8]
– Blood Cancer Patient

*"It took three visits for a third GP to send me for a blood test/chestx-ray – I had been given antibiotics for flu-like symptoms by two previous GPs. The blood test found my WBC were very high."*[9]
– Blood Cancer Patient

Diagnostic practices can be further improved through the development and encouraging uptake of national standards (Optimal Care Pathways and clinical guidelines). This is covered in Section 2.

## CASE STUDY – Katrina's five-year delay in diagnosis

When the skin on her hands began to harden and split, the mother-of-four went to her doctor and embarked on a five-year quest for answers.

The 46-year-old was eventually sent to a dermatologist in [redacted] – two hours from her family home in [redacted]. Passed between doctors who struggled to find a diagnosis and with symptoms worsening, Katrina was admitted to hospital to find her organs had started to shut down.

She only got answers after a chance meeting with a rheumatologist whose wife had been researching the early stages of the type of blood cancer Katrina was eventually diagnosed with: stage four subcutaneous T-cell lymphoma (specifically Sézary syndrome, a rare form of lymphoma affecting the skin).

With a clear diagnosis, Katrina had to urgently relocate 530 km from the family home to Sydney for treatment.

Katrina's delay in diagnosis was followed by an agonising six-month wait for a drug she needed but couldn't afford – one that was eventually made available to her on compassionate grounds.

"I used to lie in bed at night and imagine I could feel my body just multiplying these horrible little cancer cells," recalls Katrina. "I'd think, 'This is another day that I haven't had any treatment,' and I'd wonder, 'The longer I wait, is this going to give me less of a chance of a good outcome?

"If we had national standards, it would make a huge difference because some people pass away without a diagnosis. Blood cancer doesn't care what gender you identify as, what race you are or how much money you have – it impacts all walks of life, and everyone has the right to good healthcare."[10]

RECOMMENDATIONS

2. Develop initiatives such as community engagement and education on signs and symptoms of blood cancer.
3. Develop symptom support tools for GPs aimed at improving prompt and accurate diagnosis and appropriate referral to specialists of low survival, non-screenable cancers, including blood cancers.
4. As identified in the *National Strategic Action Plan for Blood Cancers* (NAP action 2.2.1), review existing international diagnostics guidelines in blood cancer and develop Australian-specific guidelines for minimum and recommended testing requirements for different types of blood cancer in Australian healthcare settings. These guidelines should be integrated with clinical guidelines.
5. Ensure Australia keeps pace with international advances in the development of new diagnostic tools.

### 1.1.3 Accessing diagnostics – Timeliness and availability

Tests can be complex and take several weeks to complete, delaying time to treatment. These delays can be increased for rural, regional and remote patients.

For example, the reporting time range for FISH (fluorescence in situ hybridisation) has been previously calculated at one to 56 calendar days. FISH testing for myeloma has recently been reported as regularly taking up to six months.[11]

Some diagnostics are not sold in Australia. Many diagnostics recognised internationally as standard of care for patients with blood cancers are not MBS-funded, meaning they are out-of-reach for many.

As blood cancers are complex to treat and have high mortality, an accurate and timely diagnosis is critical. It enables choosing the most appropriate treatment and beginning as soon as possible.

A delayed diagnosis can also increase costs:

> *"The patient took over 3 years before a diagnosis was made despite presenting with enlarged lymph nodes (ultrasound but no biopsy). So those medical costs, all the CT and biopsies and testing, and medical visits pre-diagnosis was borne out of pocket. Should have been treated at stage 1 not stage 4."*
> – Medical practitioner, State of the Nation survey[12]

Some of these barriers are similar to treatment barriers – see Section 2.1: "Barriers to treatment."

### 1.1.4 Genomic testing is expensive for patients

Many genomic diagnostic services are not publicly subsidised. This privately funded model not only limits uptake, but also means that uptake is inequitable.[13]

As a result, the option to access genomic diagnostics in Australia is limited,[14] their usage is inconsistent across the population, and ultimately few patients are undergoing genomic testing.[15]

This inequity is well-recognised:

"*Regarding genomics, we will need to ensure it can be made accessible to everyone*"[16] – Blood Cancer sector stakeholder

*"We know that genomic testing can inform treatment choices, but the process for accessing genomic testing is difficult – you do not have the same sponsor support as drugs, hospital management may decide that tests are too expensive, and patients often do not want to pay out of pocket. By implication, patient access is variable, which results in sub-quality treatment."*[17] – Blood Cancer sector stakeholder

'Equity of access' was also one of the first items discussed at the recent inaugural meeting (August 2023) of the Expert Advisory Group established to advise the Australian and state and territory governments on the impending establishment of the national genomics body, Genomics Australia.[18]

This disincentivising of genomics testing is despite growing recognition of its importance to blood cancers (and other cancers):

- WHO recently (July 2023) identified genomics as the most important emerging technology and scientific innovation: **"application of genomics for early diagnosis and pre-diagnosis of diseases**...to enable a precise diagnosis and to guide management and treatment.[19]
- The Novel Therapies Parliamentary Inquiry found "...affordable access to genomic testing is needed **not only for patients but for the future of Australia's health system**."[20]
- Our recent *State of the Nation 2023* report contains new data showing 31 per cent of blood cancer patients who had a genomic test had their diagnosis and treatment plan altered.[21]
- In a recent recommendation, the Medical Services Advisory Committee (MSAC) provided landmark support for next-generation sequencing 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'
    - 'without genetic testing, patients may be incorrectly diagnosed and potentially receive ineffective or incorrect treatment'[22] – *MSAC, March 2023*

MSAC recently (March 2023) also supported MBS funding for minimal residual disease (MRD) testing, noting a 'modest financial impact' to the MBS. Interestingly, the summary document notes that, because patients are currently paying out-of-pocket or seeking public hospital funding via the PBS to access leukaemia drug blinatumomab, it can *correct* existing inequity:

- "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**"[23] – *MSAC, March 2023*

*"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' clinician 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' clinician respondent

RECOMMENDATIONS

6. Genomic testing (precision medicine) should be embedded as the standard of care,[24] through greater public subsidy, investment in diagnostic laboratories and their

workforce, pathways for genetically trained pathologists and encouraging initiatives that support Australia's scientists undertake genomics study.

# 2 Barriers to accessing appropriate treatment

## 2.1 Barriers

### 2.1.1 Inconsistent access to best practice treatment informed by formalised standards

New Leukaemia Foundation modelling shows 29 per cent of blood cancer deaths can be avoided through the consistent adherence to national standards of timely and accurate diagnosis, treatment, and care.

This means nearly 3,000 lives of blood cancer patients could be saved every year by implementing consistent treatment standards.[25]

The reason for the variation is largely due to critical existing gaps in the current availability of Optimal Care Pathways (OCPs) and clinical guidelines for different blood cancer subtypes, and in the use of the limited number that exist.[26]

As reflected in the NAP, clinical guidelines and OCPs can minimise variation and promote best practice care. They are the foundation for achieving best practice and reducing disparities in survival outcomes. They are particularly valuable for blood cancers given many are rare and there is less information currently available than other more common cancers.

Unwarranted variation in care is beginning to be addressed by the recent introduction of blood cancer specific OCPs. OCPs make wide ranging evidence-based recommendations for best practice care, from the point of diagnosis, through treatment, survivorship and end-of-life care.

The Blood Cancer Taskforce has also been separately supported to develop a pilot clinical guideline for one blood cancer sub-type.

Further implementation of this standard-setting work currently underway will assist reduce variation in care.  Minimising discrepancy between the primary and tertiary care systems will also be assisted by embedding current best practice (e.g. through adopting OCPs) across all levels of care.

## CASE STUDY – Sarah's story (Part 1)

"In late 2020 my 23-month old son was diagnosed with a rare form of leukaemia – Acute Megakaryoblastic Leukaemia (AMKL), a sub-type of Acute Myeloid Leukaemia.

*Limited clinical knowledge and treatment options*

Knowledge of AMKL was minimal.

We were put on one treatment pathway but that failed to put our son into remission. So did the second attempt.

We then faced endless delays due to having to get approvals from the hospital board and drug companies themselves to try different cancer drugs. Many of them were for AML in adults (AMKL is almost exclusively juvenile I believe) which then had to be crushed so that they could be given to Benji through his NG line as no 2-year-old could ever hope to swallow that many (or any) chemo pills. These would then clog his line requiring it to be replaced, which was traumatic for all involved.

There was **no specific treatment protocol for his sub-type.** When the initial chemo failed to achieve remission, we were left with limited options. A further round of high dose chemo immediately after (not recommended), to be followed by a bone marrow transplant. When that chemo failed too, our only options were experimental drugs not signed off for use in children. Our options were: 1) do nothing and he dies, 2) try this and he dies, 3) try this and it kills him before the leukaemia.

While on the ward we met two other families whose children had also been diagnosed with AMKL, and **all three of us followed different treatment protocols**, There was no standard, it seemed to be based on what the consultant know and who they consulted with.

*Logistical and systemic complexities*

The chemo drugs which we were told were his last-ditch effort and had to be specially approved and ordered direct from the supplier in Sydney failed to materialize. They didn't make that evening's cargo flight from Sydney to Perth. Seven days it took for it to be realized they were never arriving and had to be reordered.

There was no match found for him on the Australian Bone Marrow Donor Register."

** Edited – For Sarah's full story, see Appendix B.*

### 2.1.2 Location and financial capacity impede access to newer therapies

Patients in regional/remote areas sometimes struggle to access the novel and specialised therapies that can be so important to treating rare and less common blood cancers.

This is because many newer diagnostics, treatments and therapies are available only through either clinical trials, which are mostly available at select major metropolitan treatment centres, or at the patient's own expense, meaning those on lower incomes can be excluded.

Similarly, those who cannot afford to access non-PBS treatments, even in metro areas, have limited access to those potentially life-saving treatments.

Research demonstrates that this contributes to potentially preventable loss of life, poor quality of life, and inequitable outcomes across patient groups.[27]

This is exacerbated by the fact that many Australians, but particularly those in rural Australia and those with lower incomes, can also struggle to be able to physically and financially access specific services. Blood cancers remain among most costly to treat, at almost triple the cost of other cancers. Around 43% of blood cancer patients report out-of-pocket expenses, and more than one in three of these incur more than $A5,000 in cost. [28]

These access gaps may be magnified by new, high-cost cellular therapies such as immunotherapies.[29]

These geographic and financial barriers can compound for rural patients. As a result, the National Action Plan shows people living in regional areas were 17 times more likely to report locational and financial barriers to care than people living in metropolitan areas.[30]

This is reflected in the experiences of many blood cancer patients, as identified through *State of the Nation 2023*:

> *"We were forced to sell our home in NSW and move to Victoria where we have direct access to a haematologist from Peter Mac and other specialist services. The WM has affected my eyes so I see an ophthalmologist and the PN has affected my bladder so I can also see a urologist locally."*[31]

> *"Treatment needs to be more accessible in regional hospitals. Travel to cities puts a huge strain on families."*[32]

> *"Need access to specialists closer to home. Travelling such vast distances takes a large toll on already fragile patients."*[33]

> *"I feel second class in regional NSW regarding accessing best treatments and trials."*[34]

Moreover, the new National Medicines Policy 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."[35]

### 2.1.3 HTA requirements delay access to treatments for rare/less common cancer

Current restrictions around the use of evidence in Health Technology Assessment (HTA) processes are delaying access for patients. Delays to the approval of new innovative medicines are of particular concern for the range of rare blood cancers with small populations.

A lack of evidence of benefit, or uncertainty in the evidence, may prevent the listing of a new medicine, device, or service on the PBS or MBS.

Uncertainty in evidence increases as the volume of sample data decreases (i.e. fewer patients). Many blood cancers are rare, and therefore uncertainty of benefit is often high.

However, smaller patient populations challenge traditional evidence generation models.

Conducting traditional randomised controlled trials is consistent with best practice, evidence-based medicine, but smaller patient populations mean trials are more difficult to recruit for.

Smaller populations can also reduce pharmaceutical company incentives to seek PBS or MBS listings for new medicines, devices, or services and in turn, could impede patients' access.[36]

Further, rapid advances in genomic profiling are enabling even more accurate subtype diagnosis.

Lack of evidence to support public subsidy – and/or a clear pathway or incentive for listing – can also have the unintended consequence of increasing 'off-label' prescribing. This can lead to inadequate generation of the evidence required to support listings, with outcomes not always reported to relevant clinical registries (e.g. bendamustine, indicated for lymphoma and CLL, is used off-label for myeloma).[37]

As a result, many potentially life-saving therapies available overseas are not available in Australia. **Appendix A** identifies blood cancer treatments and diagnostics used overseas but not in routine clinical practice in Australia.

Patients have expressed their continued frustration and desperation at not being able to access therapies and trials:

*"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.*

*Clinical trials for RRMM in Australia are few and far between, and oligo and nonsecretory myeloma patients are excluded from these trials as they do not have easily measurable disease markers (ie blood indicators).* ***Being told 'there's nothing else we can do' is tough****, especially when you know that there is, just that it's only there for the wealthy few who could afford to travel overseas and pay for it."*
– Blood cancer patient, LF Consumer Engagement Group 2023

*'It is frustrating when I know there is a better medication for my PV but it is not available in Australia. Besremi is readily available overseas. Why do we always have to wait for the best treatments?'*
– Blood cancer patient, State of the Nation 2023

*It's very stressful knowing this drug is available overseas and patients are having excellent results from it, however, I can't access it in Australia because of the cost.*
– Blood cancer patient[38]

*I travelled to Germany to take part in a clinical trial for a new leukaemia drug. It cost us $100,000, which we paid for by fundraising. After three years, I'm still disease free. It saved my life; I had no other option, but wait to die.*
– Blood cancer patient[39]

*As a 'rare cancer patient' I was given an incorrect prognosis and incorrect treatment advice on my first visit to a specialist in a regional area…it has become very obvious that many rarer cancers (and not so rare cancers) do not get enough media and government attention."*
– David, rare cancer patient

### 2.1.4 Health system fragmentation

As the *National Health Reform Agreement (NHRA) Long-term Health Reforms Roadmap*, signed by the Commonwealth and all States and Territories, acknowledges:[40]

- "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"[41] (emphasis added)

Initiatives such as the HTA Review and NHRA Review are welcome, but these reviews cannot solve these issues alone.

Relevant documents, reports, and data related to HTA processes are often not easily accessible to the public (inclusive of complex terminology), which limits stakeholder involvement in already small patient populations.

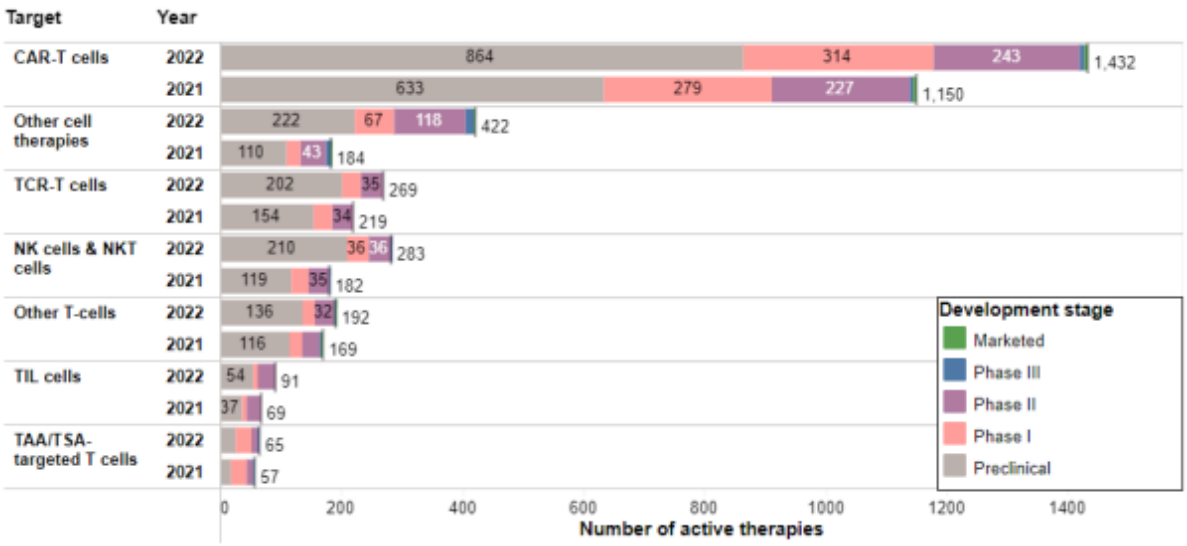
The high cost of many of these treatments often leads to negotiations between pharmaceutical companies and reimbursement bodies to determine the reimbursement amount. In some cases, the costs are shifted to other areas of the healthcare system, such as hospitals or insurance plans, to accommodate the 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.[42]

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.[43]

There is a significant pipeline of cell and gene therapies being developed globally, ultimately attempting to enter mainstream clinical practice. This is good for patients, but our fragmented system is not ready.

*Figure 1: A wave of CAR T-cell therapies on the horizon*[44]




*'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*

### 2.1.5 Workforce

Improving access to therapies, and being able to properly care for complex blood cancers, necessitates ensuring we maintain 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."[45]

This 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.[46]

Patients living outside metropolitan centres in particular are restricted in their access to practitioners.

*"Cancer patients in regional areas do not have the same choice in specialists as city patients"*

*"Some regional areas are completely without GPs."*[47]

*"The workforce remains one of the most fundamental problems. We cannot move the CAR-T space forward without having all levels of workforce developed."*[48]

### 2.1.6 Inadequate diversity on the stem cell donor registry

Not enough suitable Australians are registered as a stem cell 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.

Stem cell (bone marrow) transplants are relatively unique to blood cancer. Patients receiving transplants are required to spend substantial time in hospital and stay nearby post-transplant.[49]

Every year, more than 600 Australians with blood cancer will need donated stem cells for a potentially life-saving allogeneic transplant.

Relying on overseas donations also means patients from various ethnic backgrounds can find it even more difficult to find a suitably matched donor. Aboriginal and Torres Strait Islander people are already less likely to find a matched stem cell donor on the Registry, and have very few options overseas.

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

In February 2023, the Federal Health Minister underscored some of these challenges, noting 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."[50]

## 2.2 Potential solutions

### 2.2.1 Support for development of OCPs and their implementation

With the assistance of the Commonwealth Government, the Blood Cancer Taskforce led the development of OCPs for:

- multiple myeloma;
- chronic myeloid leukaemia ;
- chronic lymphocytic leukaemia;
- low grade lymphomas;
- childhood, adolescent, and young adult acute leukaemia; and
- myelodysplastic syndromes

These OCPs were developed to expand on the blood cancer OCPs previously developed for chronic lymphocytic leukaemia and Hodgkins and diffuse large B-cell lymphomas by the Cancer Council.

There is a critical need to address gaps in OCPs and clinical guidelines for different blood cancer subtypes. OCPs and clinical guidelines are interdependent, and to be effective, should be fully integrated into clinical practice.

RECOMMENDATION

7. A national approach to the development and sustainable management of OCPs and clinical guidelines.

### 2.2.2 Right to Trial

The Leukaemia Foundation proposes the creation of a 'Right to Trial' program. It would aim to support systematic evidence development and provide a mechanism for the more systematic evaluation of off-label use and re-purposing of drugs.[51]

The House of Representatives' *Inquiry into approval processes for new drugs and novel medical technologies in Australia* supported the Leukaemia Foundation's recommendation 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)."

A Right to Trial program could enable:

a) *Improved access* – access to new therapies and building evidence about their effectiveness in a documented, regulated clinical trial setting. Potentially this could be used as the basis for a submission or a larger clinical trial.

b) *Support for submissions* – more systematic evidence development and clinician-led or patient-led submissions for new therapies. This would provide a mechanism for the more regular and systematic use and evaluation of off-label medicines, and could reduce

dependence on industry to conduct the research needed to advance potentially curative therapies.

The Right to Trial Program would need to be developed to ensure that therapies accessed through the program met required eligibility criteria, such as the criteria used to determine off-label use, or applications for compassionate access, where safety criteria can be prescribed and met, and are in routine use already today.

In addition, there would need to be clear entry and exit timelines to limit potential unintended consequences vis-a-vis the PBS. This would provide a more systematic and scientific mechanism for the evaluation of medicines that are used off-label and more equitable access to emerging therapies.

Such a program, properly designed, would reduce inequities of access to therapies where evidence is in development.

Moreover, while the program could be piloted for blood cancers it could easily be extended to a wider range of conditions over time; it need not be blood cancer specific."[52]

RECOMMENDATION

8. The creation of a 'Right to Trial' program to support systematic evidence development and provide a mechanism for the more systematic evaluation of off-label use and re-purposing of drugs.

### 2.2.3 Additional reimbursement pathways

Australia should broaden and add to its reimbursement pathway options.

Australia has a managed entry scheme for drugs where the extent or value of the clinical effect is uncertain, but this is not well-used.[53]

Additional pathways could include:[54]

- pathways that allow for the use of earlier and more varied clinical data, including international data such as through Project Orbis. PBAC could consider and recommend reimbursement for a product based on early results, such as the ZUMA-1 or CARTITUDE-1 study, and then revise the rate of reimbursement based on updated results.
- rolling review of confirmatory clinical trials or real-world data
- increasing use of international data and real-world evidence.

For example, the National Institute for Health and Care Excellence (NICE) in the UK has a 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."

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.

Their new *Health Technology Evaluations: The Manual* states:

- "In these specific circumstances [**children, rare diseases and innovative treatments**], 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."[55]

RECOMMENDATIONS

9. That Government, subject to the outcomes of the HTA Review, identifies reimbursement pathways that allow for the use of earlier and more varied clinical data.
10. Through the Australian Cancer Plan or other instruments of government, enhance efforts to address the access challenges to new treatments and care for rare and low survival cancers.

### 2.2.4 A harmonised, national approach to cellular and genetic therapies

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.

RECOMMENDATION

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

### 2.2.5 A national, coordinated funding effort for stem cell donations

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

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.[56]

Similarly, one of the six Strategic Objectives in the draft new Australian Cancer Plan (ACP) is "Achieving Equity in Cancer Outcomes for Aboriginal and Torres Strait Islander People."[57]

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."[58]

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

12. The Inquiry considers the current funding arrangements for stem cell donations and transplants and identify opportunities to harmonise these across all jurisdictions.

# 3 The adequacy of support services after diagnosis

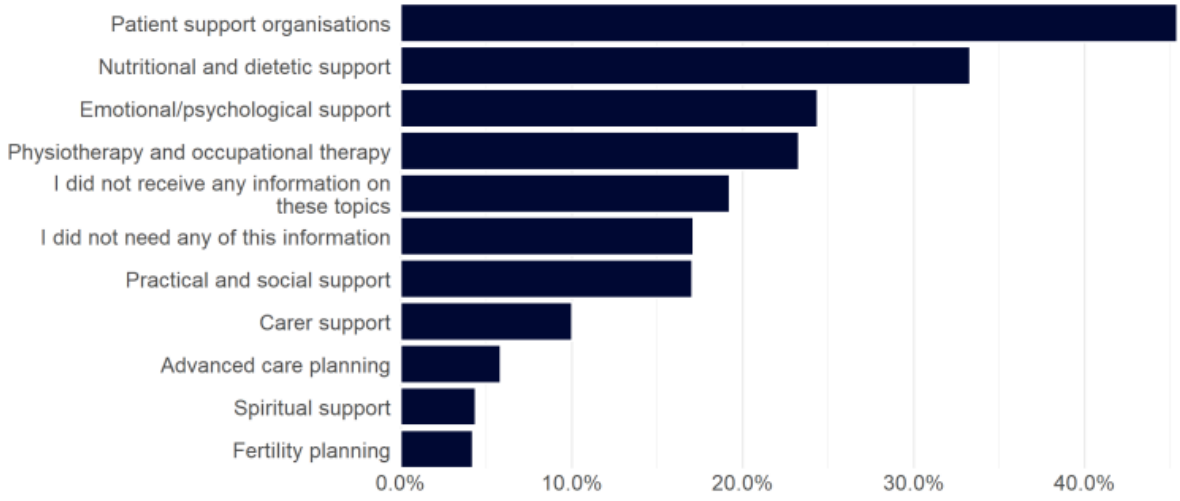
## 3.1 Patients want and need more support

Supportive care refers to the services, information and resources patients may need to meet their physical, psychological, social, information and spiritual needs from the time of diagnosis. Its benefits are well established, it is a critical aspect of cancer care, and includes referral pathways to community support organisations.[59]

Yet the Leukaemia Foundation's survey of over 4,600 Australian blood cancer patients found more support is needed across treatment planning, active treatment and post-treatment stages.

Figure 2 identifies the supports those patients wanted, with the top category being 'referrals to patient support organisations':

*Figure 2: Areas where more help was required*



One in four patients also did not know where to go to ask for help.[60]

RECOMMENDATION

13. Leverage the educational, navigation and supportive care resources of disease-specific supportive care organisations such as the Leukaemia Foundation to fast-track the delivery of supportive care.

## 3.2 Awareness and referrals are lacking

Referrals to supportive care are critical but lacking.

This is reflected in limited patient awareness about the availability of peer and nurse support, accommodation support, and transport support (e.g., from government and toll road operators).

*"The Social Worker told me that there was no help with transport for Leukaemia. This was very upsetting when I found out there was help.* [61]

*"I was lost and confused at first, I was not aware the Leukaemia Foundation could assist or help me"* [62]

*"Even after my treatment I am still finding out things I did not know, e.g. things I was eligible for but not aware of."*[63]

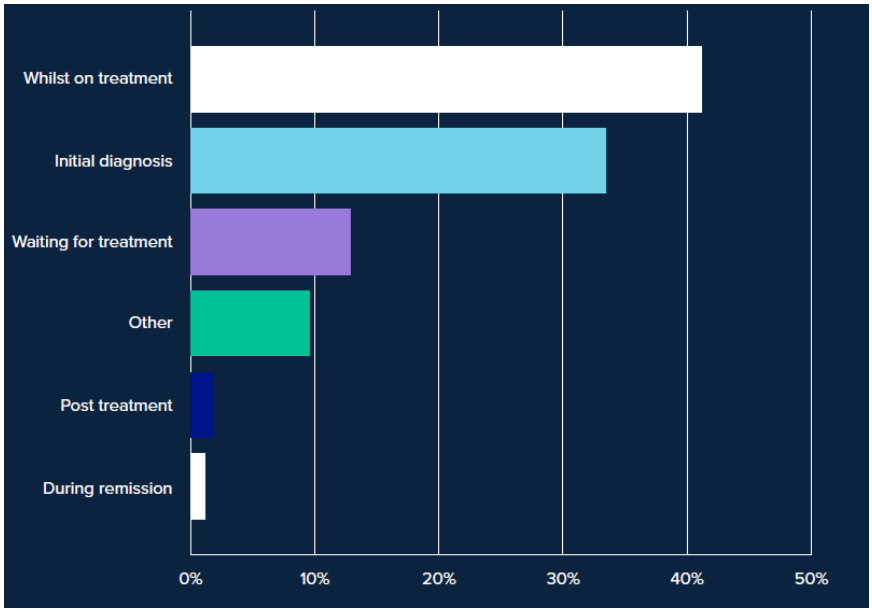
*"When my legs became swollen, it would have been helpful to be directed to or given information on what would have helped with this side effect. We found out about lymphatic massages through friends."* [64]

*"There needs to be one simple go to point. When you're diagnosed, there is so much coming at you, it's overwhelming. You need support on who to go to for different things."* [65]

In addition to practical supports with bills, accommodation and transport, the Leukaemia Foundation provides a range of resources from booklets and factsheets to seminars and education sessions, and emotional support and assistance with our highly trained support services team.

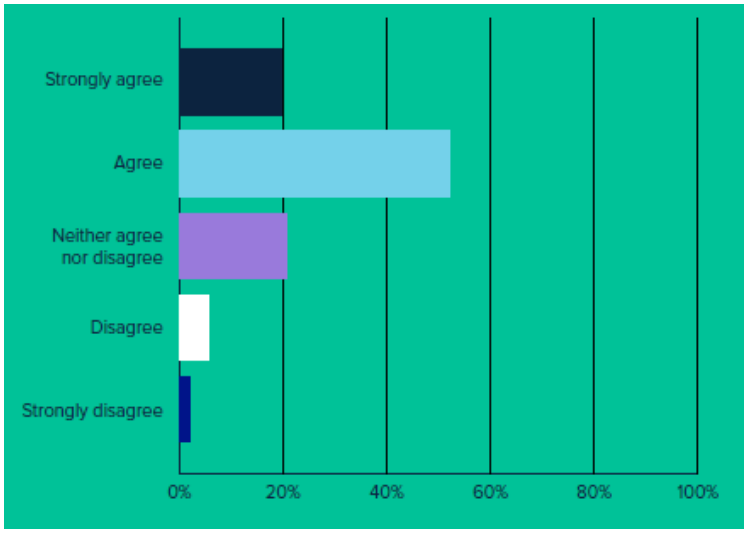
Yet the Leukaemia Foundation's July 2023 "Voice of Customer" service user survey received over 300 responses and shows only one-third were told about our services at diagnosis. This is despite diagnosis being an important time for treatment decisions and a very stressful time for patients:

*Figure 3: When did respondents first hear about the Leukaemia Foundation*




Not being told about services at diagnosis is also despite the demonstrated benefits of accessing the Leukaemia Foundation's services. For example, almost all patients felt, as a result of our support, that they could better navigate healthcare systems:

*Figure 4: Ability to navigate health care systems (after receiving LF support)*




RECOMMENDATION

14. Integrate community-led disease specific supportive care organisations such as the Leukaemia Foundation for blood cancer into the model of care.

## 3.3 More travel and accommodation support are needed

Patient Assistance Travel Schemes (PATS), operated by states and territories, provide limited and variable support.

Eighteen per cent of *State of the Nation* respondents who needed transport and accommodation support were unable to access it.[66]

One researcher noted limited support for switching carers and larger families particularly impacts Indigenous patients:

> *"The patient and transport scheme just doesn't work for Aboriginal people – having to be away for extended periods without ability to switch supporter just doesn't cater for their needs. Everyone knows that there's a problem in provision of travel assistance, and it has been a problem for years."*[67]

Some PATS do not apply to people undergoing clinical trials. For example, trials patients are unable to access the Victorian Patient Transport Assistance Scheme (VPTAS) to assist with transport and accommodation costs. The Guidelines state:

- "You are not eligible for VPTAS if you…are participating in a clinical trial or experimental treatment that aims to find a better way to manage a particular disease (p.2)."[68]

Trial access is important because, as many blood cancers are rare or less common, there are often no other treatments available. Being in a clinical trial is often a patient's last line of treatment. It is not just about "better managing" a disease – it is about staying alive.

> *"PATS does not cover trials so I can't afford transport accommodation and rest of bills."*[69]

Conversely, patients in metro areas are not eligible for many of these supports at all, as PATS generally require patients to live further than 100km. This is problematic for those undergoing expensive and arduous blood cancer treatments, such as stem cell treatments, where patients are required to stay within certain time periods from the treatment centre (e.g. within 30 mins during the first 80-100 days after transplantation).[70]

> "*Because we live less than the required 100km (90 km) from the hospital post [stem cell] transplant but are required to live close to the hospital for follow-up tests and treatment, we do not qualify to receive patient transport and accommodation rebates. This meant we've paid more than $10,000 which is a big expense for an aged pensioner."*[71]

In addition to diagnosis and treatment costs, the financial burden is compounded by ancillary effects of treatment and being away from home – often required for intensive blood cancer treatments. Again, this is particularly pertinent for blood cancers, for which the treatment can be intensive:

> *"As a single woman in her own house with no income or superannuation, expenses have made it impossible to live."*[72]

> *"My illness is chronic and doesn't get much understanding because most people ache and get tired. But it never stops. I struggle daily but I'm not sick enough for any financial assistance so I have to work.* ***There is not much understanding of blood cancers*** *when compared to breast cancer.*
>
> *I have bad symptoms (as bad as fibromyalgia), however, someone with fibromyalgia can get government assistance. It's really unfair."*[73]

> *"Having no income and still having bills to pay."*[74]

> *"My main costs were having to be 500 km from home but still having to pay my rent, electricity and water accounts as well as fuel and food for my daughter and me. It was like keeping two houses while we were away."*[75]

We know that provision of these services has positive effects for government as well as patients. Separate research by Insight Economics has found:[76]

- The total benefit to government arising from the Leukaemia Foundation's accommodation service is estimated to be $148.3 million over the 2015 – 2040 period.
- The Leukaemia Foundation's accommodation services enable at-risk families to overcome significant barriers to blood cancer treatment.
- Without access to Leukaemia Foundation accommodation:
    - 5% of families would have declined treatment
    - 10% would have sought less optimal care closer to home, likely leading to poorer outcomes
    - Roughly 80% would have exhausted their savings and incurred higher out of pocket costs if they had to self-fund their accommodation.

## SARAH'S STORY – Part 2

We were on a family holiday in Perth – myself, husband and our 3 children ranging in age from 5 to 23 months. We live remotely on [redacted], which only has 2 flights a week to Perth.

As a remote family we were eligible to stay at the Perth charity centre after our son was admitted to a [redacted] in October 2020; he would never leave again, dying there in January 2021.

### *Difficulty finding fit-for-purpose services*

According to the oncology ward's social worker who was assigned to us, we needed to find a house to rent as the charity centre was "not for people like you".

I was never entirely sure what she meant by that, and we stayed there until his death anyway - lucky, as otherwise the $75 per night from the [redacted] Patient Assisted Travel scheme (PATS) for renting we would've relied on doesn't come close to covering the costs.

We still faced disadvantages - they are not germ-controlled environments and are terrifying for a cancer parent. It is an amazing resource but it does not allow separation for families isolating from children and families with children being treated for ingrown toenails who don't care who they are sneezing and coughing on.

When we were scheduled to have a bone marrow transplant and then applied for and were granted use of one the Leukaemia Foundation's apartments which would have allowed us that isolation, but unfortunately were not able to take up the use in the end as things moved too quickly.

### *Difficulty finding support services*

In addition, because our son did not follow "the usual" and respond to treatment enough to achieve remission and a few extra months, **we seemed to be outside the scope of many support services. I don't even know if they exist** because I don't know about them and have never been able to find anything out.

Benji's sisters who were just [redacted] and [redacted] when he was diagnosed and when he died, not only missed out on schooling but **we were also not able to access any support for them**. We did not meet the criteria for support from other organisations, which appeared to be because our son died too quickly.

We received a couple of follow up phone calls from our social worker (not the original one who made the suggestion listed above) and one of the hospital play therapists, one of whom sent out a bereaved siblings support pack but for whatever reason we did not receive until 11 months after our son's death.

Organizations we have received ongoing support from include Leukaemia Foundation, Redkite, Solaris Cancer Care (WA based) and Kids Cancer Support Group (KCSG) (WA based).

Imagine if treatment like [siblings of child cancer patients automatically received access to play therapists as part of a "Whole family" approach] was funded for remote families such as ours. Although we have managed to access and pay for play therapist specializing in bereaved children for our own surviving children, each time we leave the island to see her it costs us approximately $3500 for return airfares for 2 children and 1 adult."

***Edited – For Sarah's full story, see Appendix B.***

RECOMMENDATIONS

15. Consult with all jurisdictions to reform PATS, including advocating for streamlined administrative processes, greater access to the schemes and PATS support for patients to participate in clinical trials (NAP recommendation 2.7.1).
16. Review options for the introduction of financial support mechanisms (such as a temporary disability payment) to support people with cancer and other serious illness who require temporary financial support (NAP recommendation 2.7.2).

# 4 The adequacy of Commonwealth funding for research

## 4.1 The opportunity

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.[77]

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.[78]

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."[79]

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

Increasing amounts of datasets, fueled by big data and precision medicine, are emerging as a powerful research tool. The National Clinical Quality Registry Strategy is an opportunity to build data linkage, integration and interoperability capability.

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

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).[80]

Finally, 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.[81]

## 4.2 The challenges

### 4.2.1 Blood cancer research is underfunded

Blood cancer research is relatively underfunded, with blood cancers such as lymphoma and myeloma receiving relatively low funding support.[82]

We also currently know there are over 120 discrete blood cancers. As our understanding of the genetic basis of blood cancer improves through research, the number of identified blood cancer subtypes is increasing.

This “splitting” into smaller and smaller subtypes can challenge traditional approaches to research, to the cost-effective assessment of new therapies and creates a new imperative for strategic research collaboration for impact.

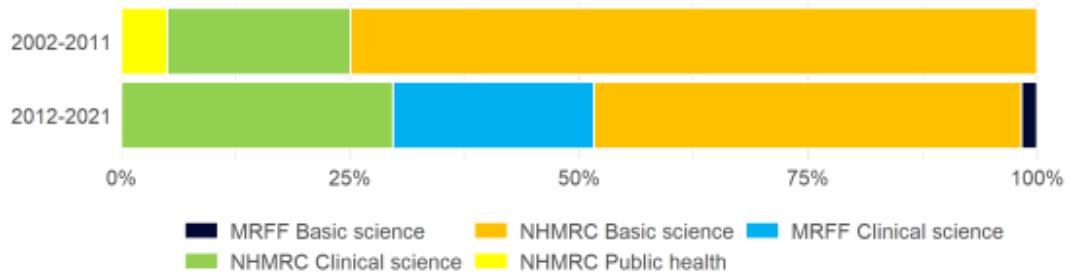
In addition to supporting an increased quantum of research for blood cancer research, this submission makes specific recommendations relating to uncommon and rare cancers relating to evidentiary requirements for health technology assessment for cell and gene therapies (Section 2).

> *"There seems to be an assumption that childhood leukemia has had a “cure” found, at least according to the media, but I have also seen such proclamations from child cancer charities and their patrons. I watched my son die 104 days after his diagnosis – and he was diagnosed very early on (according to his consultant)."*

### 4.2.2 Further support is required for basic research

Government funding for basic research has declined from 75 per cent during 2002-2011 to only 48 per cent during 2012-2021:[83]

*Figure 5: NHMRC and MRFF funding for clinical and basic sciences, blood cancers*




More broadly, Australia’s R&D sector funding (1.8% of GDP) is well below the OECD average (2.67%).[84]

### 4.2.3 Workforce challenges

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."[85]

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."[86]
- Attrition of the clinical trials workforce is **impeding research activity.**[87]
- "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."[88]

Last year, only 11% of the NHMRC's Ideas Grant applications were funded. Only 15% went to early- and mid-career researchers.[89]

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.[90]

These challenges are compounded by a pre-existing STEM (science, technology, engineering and mathematics) skills and teacher shortage.[91]

### 4.2.4 Further support for translation and collaborative research is needed

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.[92]

The national and international collaborations over several decades that led to Venetoclax being available to patients underscores the importance of partnerships and supporting research 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.[93]

In line with this desire for a collaborative approach, the Leukaemia Foundation is funding the development of a Research Roadmap, to map out a 10-year plan to develop and 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.

### 4.2.5 Clinical trials

The OCPs reinforce that 'clinical trials are the foundation for improved cancer outcomes,' and that they may also be 'a valuable option for people with rare, difficult-to-treat conditions for which there may be limited evidence.'

The OCPs also note the care team 'should support the patient to participate in research or clinical trials where available and appropriate, regardless of geographical location or ethnic or linguistic background.'[94]

The 2022 Survey of People Living with Blood Cancer found that only 21 per cent participated in a clinical trial, and that among people who did not participate in a clinical trial the primary reason was that it was not discussed (74% of respondents), while a further 10 per cent indicated that their specialist had looked for a trial but no relevant clinical trial was available.

RECOMMENDATIONS

17. Australia's research funding 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.
18. Define new investment in such areas as genomics, microbiota, diagnostics, immunotherapies, targeted therapies and cellular therapies which hold the potential to address the unmet needs of blood cancer subtypes for which there is currently no cure and where five-year survival is poor.
19. Develop a research strategy for low survival cancers, including emphasis on development of transformative therapies and a targeted support framework to execute this.
20. Through the implementation of the National Clinical Quality Registry Strategy, a review should be undertaken to evaluate options to improve the value and sustainability of blood cancer registries and Australian blood cancer patient data by extending the coverage of these registries. This should leverage from, and link to, existing administrative datasets and annotated samples stored at related biobanks, and enable linkages with international real world data endeavours.

# 5 Other

## 5.1 Alignment with other plans

This Inquiry should ensure recommendations align with government-supported strategies being released shortly, or identify gaps relating to rare cancers in those strategies.

The first national **Australian Cancer Plan** (ACP) is an important cancer blueprint. We support the draft Plan's focus on inclusion, equity and hard to reach and/or underserved populations, which relate to this current Inquiry.

We are concerned, however, that the capacity for the ACP to meet its ten-year ambitions for people living with blood cancer could be hampered if the 'tumour-agnostic' guiding principle is pursued in all instances, without any explicit mechanisms for addressing disease-specific challenges.

We highlighted a major gap in the draft ACP – the absence of any emphasis on developing transformative therapies for low survival cancers. Screening was a focus in the draft, but is not applicable for blood cancers.

Government has also announced possible changes to national medical research funding arrangements, and is conducting a consultation. Importantly, this will then lead into consultation on a separate **national strategy for health and medical research**.

The Aboriginal and Torres Strait Islander Cancer Plan developed by the National Aboriginal Community Controlled Health Organisation (NACCHO) is another important plan to be released in late 2023.

RECOMMENDATION

21. Ensure alignment with the upcoming ACP, national strategy for health and medical research, NACCHO Cancer Plan and other initiatives, including identifying gaps applicable to blood cancers.

## 5.2 Elevating Patient Reported Outcomes

Using PROs allows the lived experience of patients to be better reflected in healthcare decisions, but they are not adequately embedded in our healthcare system.

Specifically, they can:[95]

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

The smaller populations of rare cancer patients means this can be challenging, but PROs can provide valuable information on many of the barriers identified in this submission, including service provision, experiences of rural patients and others.

Governments, clinicians, service providers and the broader sector need to ensure PROs continue to be used and collected. This aligns with a raft of policy imperatives, including:

- The NAP called for a national system for patient reported outcomes in blood cancer, and this has been adopted in the draft ACP.
- 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."[96]

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.

RECOMMENDATION

22. That the Committee recommends development of a national system for PROs, which should be aligned to the PRO recommendations in the ACP (when publicly available).

# 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 |
|---|---|---|---|
| **ALL** | 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. | blinatumomab<br>imatinib for Ph-like ALL<br>nilotinib for Ph-like ALL<br>dasatinib for Ph-like ALL<br>ponatinib for Ph-like ALL | CAR T-cell therapy for adults (over 25)<br>Lack of options for relapsed T-ALL |
| | 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 | PBS restrictions on TKI use beyond 2 years of maintenance | |
| **AML** | Lack of funding options for advanced diagnostics (MRD PCR and flow, microarray, NGS), which may impact on transplant decisions. | 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.<br>gliteritinib (*FLT3* inhibitor)<br>IDH-1 and 2 inhibitors (enasidenib, ivosenib) | 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.<br>Cell therapy |
| **Lower Risk MDS** | Molecular testing unfunded/unavailable | Therapies that improve cytopaenias and /or transfusion dependence are largely unavailable or unfunded (e.g. EPO and luspatercept) | Therapies for relapsed / refractory MDS are nonfunded or unavailable |
| **Intermediate and / or higher risk MDS** | Molecular testing unfunded/unavailable and this may change risk and /or allotransplant decisions | Therapies for patients with 10-20% blasts are limited with CR rates<50% and /or significant toxicities for combination therapy (e.g., venetoclax) | Nonfunded or unavailable for MDS phenotype unless patient progresses to AML |
| **CML** | - | - | - |

| | | | |
|---|---|---|---|
| **CLL** | Access to gene array and mutation screening by NGS (e.g. TP53)<br>Frontline young patients no access to novel agents<br>Inequitable access of genetic testing necessary to inform prognosis | acalabrutinib ± obinutuzumab,<br>venetoclax + obinutuzumab for younger patients<br>zanubrutinib<br>ibrutinib | CAR-T cell therapy<br>Triple refractory population- gaps in drug options<br>Richter's Transformation - gaps in drug options<br>Access to venetoclax retreatment<br>Double refractory treatment options<br>pirtobrutinib |
| **Lymphoma** | Lack of funding options for advanced diagnostics (NGS 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.<br>FISH testing used to diagnose HGBL-double hit. | critzotinib (ALK+Lymphoma, histiocytosis) for first line or relapse<br>BRAF inhibitors for hairy cell leukaemia and histiocytosis.<br>Thiotepa for transplant therapy in primary central nervous system lymphoma<br>bortezomib for abc-DLBCL, and in mantle cell lymphoma<br>polatuzumab for frontline DLBCL | azacitidine (T-Cell)<br>CAR T-cell therapy (Follicular)<br>CAR T-cell therapy (Richter's)<br>CAR T-cell therapy (Mantle cell)<br>Bi-specific antibodies including mosunetuzumab in FL, glofitimab and epcoritimab in DLBCL.<br>tafasitabam and lenalidomide and polatuzumab BR for Diffuse Large B-Cell<br>PD1 inhibitors for extranodal natural killer/T-cell<br>EBV-specific CTL lines for extranodal natural killer/T-cell and other ebv-PTLDs<br>pirtobrutinib in Mantle Cell Lymphoma<br>tazemetostat in EZH2-mutated Follicular Lympohoma |
| **Hodgkin lymphoma** | | brentuximab vedotin (CD30) in Advanced stage (AAVD) | |
| **Myeloma** | CT/PET and whole body STIR MRI skeletal surveys<br>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.<br>Lack of funding options for advanced diagnostics (NGS and MRD), including gene expression profile for baseline risk stratification. | bortezomib, lenalidomide, and dexamethasone (VRd)<br>Bendumustine<br>Daratumumab-RevDex for non-transplant eligible patients<br>Specific regimens for high-risk myeloma, such as KCRD-daratumumab<br>daratumumab-VTD induction for some patient cohorts | daratumumab-Pd<br>KPd<br>CAR-T cell therapy (both idecel and ciltacel)<br>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. [97]

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# APPENDIX B: CASE STUDY – Sarah's story

"In late 2020 my 23-month old son was diagnosed with a rare form of leukaemia – Acute Megakaryoblastic Leukaemia (AMKL), a sub-type of Acute Myeloid Leukaemia.

We were on a family holiday in Perth – myself, husband and our 3 children ranging in age from 5 to 23 months. We live remotely on , which only has 2 flights a week to Perth. As a remote family we were eligible to stay at the Perth charity centre after our son was admitted to a Perth Hospital in October 2020; he would never leave again, dying there in January 2021.

*Difficulty finding fit-for-purpose services*

Firstly, according to the oncology ward's social worker who was assigned to us, we needed to find a house to rent as the charity centre was "not for people like you".

I was never entirely sure what she meant by that, and we stayed there until his death anyway - lucky, as otherwise the $75 per night from the Patient Assisted Travel scheme (PATS) we would've relied on doesn't come close to covering the costs.

We still faced disadvantages – they are not germ-controlled environments and are terrifying for a cancer parent. It is an amazing resource but it does not allow separation for families isolating from children and families with children being treated for ingrown toenails who don't care who they are sneezing and coughing on.

When we were scheduled to have a bone marrow transplant and then applied for and were granted use of one the Leukaemia Foundation's apartments which would have allowed us that isolation, but unfortunately were not able to take up the use in the end as things moved too quickly.

*Limited clinical knowledge and treatment options*

In addition, knowledge of AMKL was minimal.

We were put on one treatment pathway but that failed to put our son into remission. So did the second attempt.

We then faced endless delays due to having to get approvals from the hospital board and drug companies themselves to try different cancer drugs. Many of them were for AML in adults (AMKL is almost exclusively juvenile I believe) which then had to be crushed so that they could be given to Benji through his NG line as no 2-year-old could ever hope to swallow that many (or any) chemo pills. These would then clog his line requiring it to be replaced, which was traumatic for all involved.

There was no specific treatment protocol for his sub-type. When the initial chemo failed to achieve remission, we were left with limited options. A further round of high dose chemo immediately after (not recommended), to be followed by a bone marrow transplant. When that chemo failed too, our only options were experimental drugs not signed off for use in



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children. Our options were: 1) do nothing and he dies, 2) try this and he dies, 3) try this and it kills him before the leukaemia.

While on the ward we met two other families whose children had also been diagnosed with AMKL, and all three of us followed different treatment protocols. There was no standard, it seemed to be based on what the consultant know and who they consulted with.

*Logistical challenges*

We could only have two people in the hospital room with our son at any point in time. And our children were not allowed. So that's another hurdle. What do we do with the other children while spending time with our critically ill son? Where do they go to school?

Due to us being based on but in Perth for treatment, my husband was unable to work. As a New Zealand citizen I was ineligible to claim any carers' allowance for my dying child.

The ward didn't allow siblings to visit for any point in time, and the hospital required us to have someone with our son all the time. We did not have any family in WA to rely on for childcare or hospital stays so there was just the two of us, one in hospital, one with the other children.

We were unable to even travel home to pick up any clothes or belongings due to the fact there's only two flights a week and they are regularly cancelled, and it was approaching the wet season when travel disruption is frequent.

I washed my families clothes in the communal washing machines at the hospital for the entire 3 months (washing machines were available at the charity centre but I was never there – I was at the hospital) - I used to go up to the laundry at 3am as it was "usually" empty then, imagine there being a funded laundry service or one that families pay for so I could have spent those nights with my son instead of having to put him back in his bed so I could get up and do laundry.

The chemo drugs which we were told were his last ditch effort and had to be specially approved and ordered direct from the supplier in Sydney failed to materialize. They didn't make that evening's cargo flight from Sydney to Perth, seven days it took for it to be realized they were never arriving and had to be reordered.

There was no match found for him on the Australian Bone Marrow Donor Register.

*Difficulty finding support services*

In addition, because our son did not follow "the usual" and respond to treatment enough to achieve remission and a few extra months, we seemed to be outside the scope of many support services. I don't even know if they exist because I don't know about them and have never been able to find anything out.

Our other children, Benji's sisters who were just and when he was diagnosed and when he died, not only missed out on schooling but we were also not able to access any support for them. We did not meet the criteria for support from other organisations, which appeared to be because our son died too quickly.

We received a couple of follow up phone calls from our social worker (not the original one who made the suggestion listed above) and one of the hospital play therapists, one of whom sent out a bereaved siblings support pack but for whatever reason we did not receive until 11 months after our son's death.

Organizations we have received ongoing support from include Leukaemia Foundation, Redkite, Solaris Cancer Care (WA based) and Kids Cancer Support Group (KCSG) (WA based). Childhood trauma is such an important thing to try and resolve, I am aware of the pressures the health system is under but imagine if the siblings of child cancer patients automatically received access to play therapists as part of a "Whole family" approach to treatment.

Imagine if treatment like this was funded for remote families such as ours, as although we have managed to access and pay for play therapist specializing in bereaved children for our own surviving children, each time we leave the island to see her it costs us approximately $3500 for return airfares for 2 children and 1 adult.

*Final word as a rare leukaemia parent*

There seems to be an assumption that childhood leukemia has had a "Cure" found, at least according to the media, but I have also seen such proclamations from child cancer charities and their patrons. I watched my son die 104 days after his diagnosis – and he was diagnosed very early on (according to his consultant)."

[1] 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.
[2] National Action Plan, p.24: https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer_June-2020.pdf
[3] National Action Plan, p.34.
[4] Blood cancer patient cited in State of the Nation 2023, p.56.
[5] Blood cancer patient cited in *State of the Nation 2023*, p.13.
[6] Blood cancer patient cited in State of the Nation 2023, p.47.
[7] Blood cancer patient cited in State of the Nation 2023, p.29.
[8] Blood cancer patient cited in State of the Nation 2023, p.47.
[9] Blood cancer patient cited in State of the Nation 2023, p.47.
[10] https://www.leukaemia.org.au/stories/for-mother-of-four-katrina-richards-a-five-year-delay-in-diagnosis-was-followed-by-a-further-wait-for-access-to-treatment/
[11] *State of the Nation: Blood Cancers in Australia Report 2023, p.50.*
[12] Medical practitioner cited in *State of the Nation 2023*, p.8.
[13] *State of the Nation: Blood Cancers in Australia Report 2023,* p.84.
[14] 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
[15] *State of the Nation: Blood Cancers in Australia Report 2023,* p.85.
[16] State of the Nation 2023, p.49.
[17] State of the Nation 2023, p.49.
[18] Expert Advisory Group on Genomics Australia, Terms of Reference, August 2023: https://www.health.gov.au/sites/default/files/2023-08/expert-advisory-group-on-genomics-australia-terms-of-reference.pdf
[19] *2023 Emerging technologies and scientific innovations*, World Health Organisation, 2023: https://www.who.int/publications/i/item/9789240073876.
[20] New Frontiers report, p.229: https://parlinfo.aph.gov.au/parlInfo/download/committees/reportrep/024755/toc_pdf/TheNewFrontier-DeliveringbetterhealthforallAustralians.pdf;fileType=application%2Fpdf
[21] *State of the Nation: Blood Cancers in Australia Report 2023*
[22] 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/$File/1684%20Final%20PSD-Nov%202022.pdf
[23] MSAC public summary document: *Application No. 1707 – clonoSEQ® and mpFC for the detection of measurable residual disease (MRD) in acute lymphoblastic leukaemia (ALL);* http://www.msac.gov.au/internet/msac/publishing.nsf/Content/67D026849586C408CA25879B008371EC/$File/1707%20Final%20PSD-Nov2022_redacted.pdf

[24] 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
[25] https://www.leukaemia.org.au/media/postcode-lottery-lives-still-being-needlessly-lost-to-blood-cancer-due-to-inconsistencies-in-treatment/
[26] NAP p.33: https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer_June-2020.pdf
[27] *State of the Nation: Blood Cancers in Australia Report 2023*, p.xvii.
[28] https://www.leukaemia.org.au/media/australias-costly-cancer-burden-blood-cancer-patients-facing-impossible-choices-amid-soaring-costs/
[29] *State of the Nation: Blood Cancers in Australia Report 2023*, p.78.
[30] NAP 2020, p.3: https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer_June-2020.pdf
[31] Blood cancer patient cited in *State of the Nation 2023*, p.8.
[32] Blood cancer patient cited in State of the Nation 2023, p.38.
[33] Blood cancer patient cited in State of the Nation 2023, p.38
[34] Blood cancer patient cited in State of the Nation 2023, p.38
[35] *National Medicines Policy*, https://www.health.gov.au/sites/default/files/2022-12/national-medicines-policy.pdf
[36] Submission to *Inquiry into approval processes for new drugs and novel medical technologies in Australia*, p.5.
[37] Blood Cancer Taskforce, 2020, *Rapid Review of Evidence*, Table 5.3, pp.189-190. Available at https://www.leukaemia.org.au/wp-content/uploads/2020/09/National-Strategic-Action-Plan-for-Blood-Cancer_Review-of-Evidence-for-Action-1.pdf
[38] Leukaemia Foundation submission to *Availability of new, innovative and specialist cancer drugs in Australia*, 2015.
[39] Leukaemia Foundation submission to *Availability of new, innovative and specialist cancer drugs in Australia*, 2015.
[40] *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
[41] *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.
[42] 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
[43] *State of the Nation: Blood Cancers in Australia Report 2023*, p.78.
[44] 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.
[45] *National Medicines Policy*, https://www.health.gov.au/sites/default/files/2022-12/national-medicines-policy.pdf
[46] *State of the Nation: Blood Cancers in Australia Report 2023,* p.53.
[47] Blood cancer sector stakeholder cited in State of the Nation 2023, p.38 – 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.
[48] State of the Nation 2023, p.53 – 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.
[49] *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
[50] Hansard, 7 February 2023, https://www.aph.gov.au/Parliamentary_Business/Hansard/Hansard_Display?bid=chamber/hansardr/26418/&sid=0080
[51] LF submission to *Inquiry into approval processes for new drugs and novel medical technologies in Australia*, 2020.
[52] *State of the Nation 2020*, p. 100.
[53]*State of the Nation 2023*, p.81.
[54] *State of the Nation 2023*,, p.81.
[55] *NICE Manual 2022*, https://www.nice.org.uk/process/pmg36/resources/nice-health-technology-evaluations-the-manual-pdf-72286779244741
[56] https://www.leukaemia.org.au/national-action-plan/
[57] https://engage.australiancancerplan.gov.au/
[58] https://www.health.gov.au/sites/default/files/2023-02/health-ministers-meeting-communique-24-february-2023.pdf
[59] Optimal Care Pathway, MDS: https://www.cancer.org.au/assets/pdf/myelodysplastic-syndrome-1st-edition#_ga=2.55482412.647209155.1692431694-1182099395.1684481384
[60]State of the Nation 2023, p.34
[61] Blood cancer patient cited in State of the Nation 2023, p.36 – 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.
[62] "Voice of Customer Survey," July 2023, Leukaemia Foundation.
[63] Blood cancer patient cited in State of the Nation 2023, p.36 – 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.
[64] Blood cancer patient cited in State of the Nation 2023, p.36.
[65] Blood cancer patient cited in State of the Nation 2023, p.37.
[66] State of the Nation, p.54.
[67] State of the Nation, p.54.
[68] VPTAS Guidelines, p.2: https://www.health.vic.gov.au/sites/default/files/migrated/files/collections/policies-and-guidelines/v/vptas-guidelines-201601.pdf
[69] Blood cancer patient cited in State of the Nation 2023, p.38.
[70] Association of Distance from Transplant center and Place of Residence on Outcomes after Allogeneic Hematopoietic Cell Transplantation, https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4905774/
[71] Blood cancer patient cited in *State of the Nation 2023*, p.8.
[72] Blood cancer patient cited in *State of the Nation 2023*, p.8.
[73] Blood cancer patient cited in *State of the Nation 2023*, p.13.
[74] Blood cancer patient cited in *State of the Nation 2023*, p.8.
[75] Blood cancer patient cited in *State of the Nation 2023*, p.8.

[76] Insight Economics Pty Ltd, The Health, Social and Economic Impacts of the Leukaemia Foundation, February 2021: https://www.leukaemia.org.au/wp-content/uploads/2021/11/AnnualReport_DigitalA4_November21.pdf
[77] *State of the Nation: Blood Cancers in Australia Report 2023*, p.42.
[78] *State of the Nation: Blood Cancers in Australia Report 2023, p.50.*
[79] Victorian Cancer Registry, 2022, *Cancer in Victoria 2021.*
[80] *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
[81] KPMG, *Economic impact of medical research*, 2018, p.6.
[82] https://www.canceraustralia.gov.au/publications-and-resources/cancer-australia-publications/cancer-research-australia-overview-funding-cancer-research-projects-and-programs-australia-2012-2020
[83] *State of the Nation: Blood Cancers in Australia Report 2023,* p.65.
[84] ATSE Pre-Budget Submission, p.14: https://www.atse.org.au/wp-content/uploads/2023/01/ATSE-SUB-230127-Pre-budget-submission.pdf
[85] https://www.health.gov.au/sites/default/files/2022-12/national-medicines-policy.pdf
[86] *State of the Nation: Blood Cancers in Australia Report 2023*, p.66.
[87] *State of the Nation: Blood Cancers in Australia Report 2023*, p.66.
[88] *State of the Nation: Blood Cancers in Australia Report 2023*, p.53.
[89] Budget Proposal 2023-24, https://www.aamri.org.au/wp-content/uploads/2023/02/AAMRI_Budget_Proposal_2023.pdf
[90] https://www.aamri.org.au/wp-content/uploads/2023/02/AAMRI_Budget_Proposal_2023.pdf
[91] https://www.atse.org.au/wp-content/uploads/2022/08/SBM-2022-08-08-Jobs-and-Skills-Australia-bill-v3.pdf
[92] 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
[93] https://www.nhmrc.gov.au/research-policy/research-translation-and-impact/research-translation-strategy-2022-2025
[94] https://www.cancer.org.au/assets/pdf/hodgkin-and-diffuse-large-b-cell-lymphoma-2nd-edition
[95] 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
[96] NHRA Roadmap, https://www.health.gov.au/sites/default/files/documents/2021/10/national-health-reform-agreement-nhra-long-term-health-reforms-roadmap.pdf, p.18.
[97] State of the Nation, pp.75-77.