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**RESEARCH ROADMAP FOR BLOOD CANCER**

# 10-YEAR RESEARCH ROADMAP

TO ACCELERATE BLOOD CANCER RESEARCH  
IN AUSTRALIA

**OCTOBER 2024**







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# ACKNOWLEDGEMENTS

## ACKNOWLEDGEMENT OF COUNTRY

The Australian Academy of Science (the Academy) acknowledges and pays respect to the Ngunnawal people, the Traditional Owners of the lands on which the Academy office is located. The Academy also acknowledges and pays respect to the Traditional Owners and the Elders both past and present of all the lands on which the Academy operates, and its Fellows live and work. We recognise the continuous living culture of Aboriginal and Torres Strait Islander peoples and their diverse languages, customs and traditions, knowledges and systems.

## EXPERT STEERING GROUP

An expert steering group was formed to advise and contribute expertise to the project, while governance remained with the Australian Academy of Science.

The following representatives formed the expert steering group:

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# EXECUTIVE SUMMARY

Blood cancers are one of the most common cancers globally and are expected to become the most prevalent and deadly form of cancer in Australia by 2035.<sup>1</sup>

Incidence has risen by 47% over the past decade, far outpacing population growth,<sup>2</sup> and is expected to double in the next decade.<sup>3</sup>

The cause of this increase is unknown, but understanding it is critical to developing new treatment and management options. Blood cancers are typically non-hereditary, non-screenable, and spontaneous cancers, with more than 100 subtypes,<sup>4</sup> making detection and treatment challenging.

Past research has delivered dramatic gains in survival for some specific blood cancers (see Appendix). For those difficult-to-treat blood cancers with high mortality rates, new research is the only way to improve outcomes.

## IMMEDIATE ACTION IS NEEDED

Right now, we are losing the race. To reverse or slow the rise of blood cancer incidence, and to minimise deaths and impacts, Australia must increase its investment in research—both fundamental and applied—and the workforce that drives it.

Current funding is not sufficient to meet the existing challenge of high-mortality blood cancers, nor to avert the rising incidence.

This is a once-in-a-generation opportunity to build a coordinated and strategic approach and to pioneer new treatments for blood cancer, building on the foundation of our existing research strengths and clinical trial capability.

## THE QUEST: ZERO PREVENTABLE DEATHS BY 2035

We propose a national quest, supported by government, industry and philanthropic stakeholders, to fast-track research efforts, leverage existing infrastructure and strengths, and build new opportunities and capacity. The quest will address the overarching issues applicable to all healthcare and cancer research and is a shared responsibility of all health researchers to emphasise areas where we can make the greatest differences.

This national quest has the following three priorities:

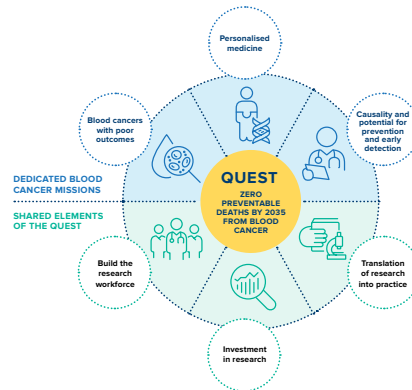
- **Investment in research:** Increasing funding for blood cancer research—including fundamental research—with dedicated funding streams to tackle areas of particular need, including difficult-to-target and high-mortality cancers.
- **Translation of research into practice:** Improving national coordination of research, incentivising multidisciplinary teamwork, enabling strategic international collaboration and partnerships, and supporting large-scale clinical trials.
- **Build the research workforce:** Bridging the gap between research and clinical care by empowering researchers and clinician–researchers with the skills, resources, and support they need to conduct research, advance health and medical knowledge, and improve patient care and outcomes.

## THREE BLOOD CANCER MISSIONS

To support the quest for zero preventable deaths by 2035 and deliver this Research Roadmap, three missions have been identified that address areas of high unmet need. Each mission offers a new focus for blood cancer research. The Australian Government must lead the investment of \$125 million into blood cancer research, spread across the three national missions (see p14).

1. **Blood cancers with poor outcomes:** We need to explore better options to manage cancers such as acute myeloid leukaemia (AML), poor prognosis multiple myeloma, recurrent aggressive lymphomas, and currently intractable rare blood cancers.
2. **Causality and potential for prevention and early detection:** This mission should focus on including early detection in populations and the small subset of individuals known to be of high risk of developing a blood cancer—from environmental influences, prior cancer therapy and heritable factors.
3. **Personalised medicine:** The third mission should focus on integrating advanced genomics, and targeted and cellular therapies in models of care, aiming to change the natural history of blood cancers.

## ACTION AREAS



### Investing in research

- Need for fundamental research into the full spectrum of blood cancers
- Need to enhance prevention, early detection and diagnostics
- Build informative and accessible research



### Translation of research into practice

- Translation transforms outcomes
- Advance personalised medicine
- Embed clinical trials
- Expanding health systems research



### Skilled workforce

- Build the researcher workforce across all disciplines and career stages



# THE BLOOD CANCER CHALLENGE

## A LEADING CAUSE OF DEATH

Blood cancers are already the third-largest cause of cancer deaths in Australia. They affect Australians at every stage of life from childhood through to old age. They are the most common cancer for children and young adults, and a major cause of death in middle-and-older-aged adults (see Appendix).<sup>5</sup>

Blood cancers in Australia are expected to double by 2035 and are predicted to become the most prevalent cancer in Australia.

Blood cancer patients represented an estimated 12% of all cancer cases diagnosed in Australia in 2023—more than 19,500 people. In 2023, approximately 51,000 people died from cancer; of these, 5,600 died from blood cancer, representing just under 11% of all cancer deaths.<sup>6</sup>

If the fatality rate stays the same and the prevalence doubles, blood cancers are on track to become one of the leading causes of all deaths in Australia.

## GROWING FINANCIAL BURDEN

Blood cancers are among the costliest cancers to treat, with myeloma and leukaemia in the top five. Costs escalate further if blood cancers recur and additional rounds of treatments are required after initial therapy. Direct health costs for all blood cancers are predicted to triple, to \$10.9 billion by 2035.

In addition to the health system costs, individual patients bear significant costs too—more than one in three report out-of-pocket costs of more than \$5,000.<sup>7</sup>

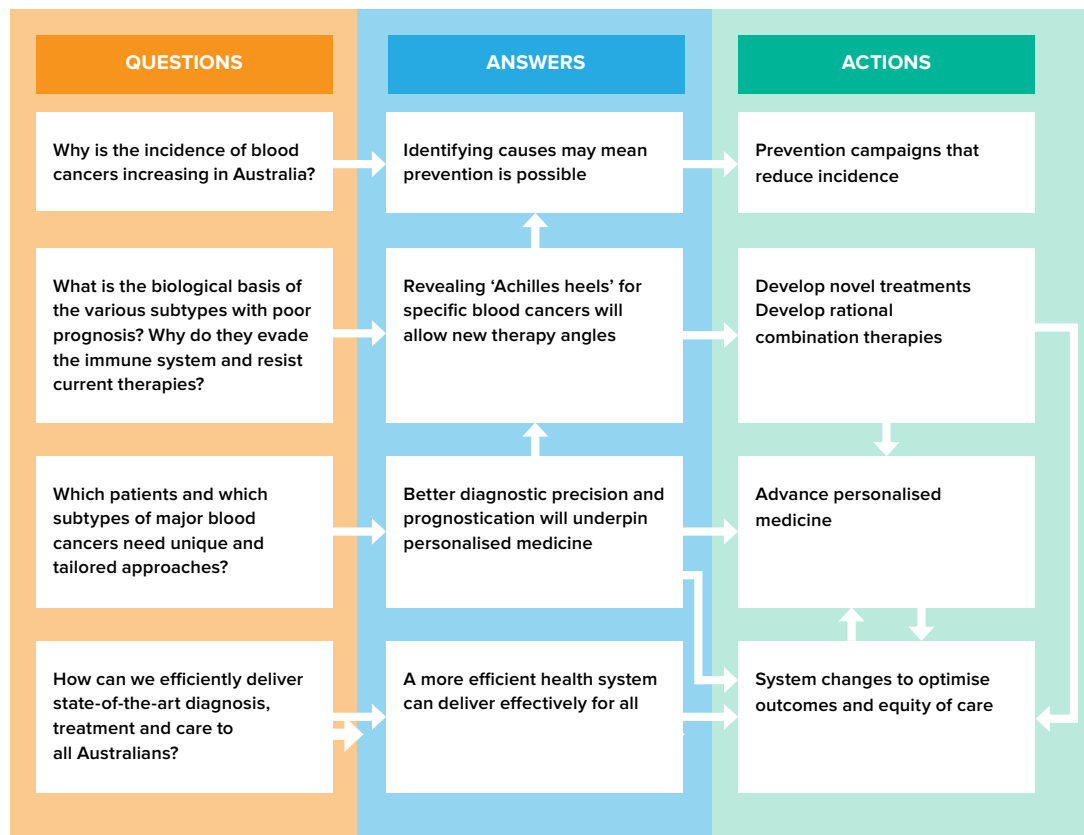
## ADDRESSING THE ISSUE FROM THE GENESIS

There is little understanding of how or why many blood cancers develop. Many cannot be prevented and there is no current national screening program. Unlike solid cancers (e.g. breast, bowel, lung), most blood cancers occur in multiple areas of the body simultaneously, reducing the potential for early detection to enable cures. Given the distinctive nature of these cancers, solutions must focus on where we can make the greatest gains.

Improved clinical outcomes will only be achieved from an uplift in research investment, which will lead to new understanding, better diagnostics and therapies, and more effective use of these knowledge advances.

## KEY GAPS IN KNOWLEDGE

Seeking answers to critical questions will reveal solutions and ultimately help reverse the predicted increase in blood cancer deaths.



## FURTHER SUPPORT IS NEEDED

Between 2012 and 2020, blood cancer research was awarded \$262.1 million of funding, representing 12% of all cancer research funding.<sup>8</sup> Although this funding is in line with current blood cancer incidence, action is required now to stem the growing caseload and save lives.

Across all funding sources (both government and non-government), funding increased from \$78.6 million in the 2015–2017 triennium to \$125 million in the 2018–2020 triennium.

The increase in funding mostly came from the introduction of the Medical Research Future Fund (MRFF) and an uptick in philanthropic funding. (The latter has subsequently declined due to the COVID-19 pandemic's detrimental effect on fundraising.)

Recent increases in funding have focused on research involving and improving upon existing therapies. Meanwhile, funding for fundamental research, 'blue-sky' aetiology research, or prevention research has not increased, despite these areas needing a greater proportion of funding (see Figure 1).

# THE IMPORTANCE OF ADVANCING BLOOD CANCER RESEARCH IN AUSTRALIA

Australia has made significant contributions to domestic and global blood cancer research and treatment. Based on publication metrics, Australian blood cancer researchers currently outperform the global average.

With some of the world's best health and medical scientists, Australia has played a pivotal role in a long list of groundbreaking discoveries. Australian researchers have also successfully translated these discoveries into transformative advances in care for specific blood cancers and selected patients. Examples that have been adopted worldwide include:

1. Discovery of G-CSF,<sup>9</sup> a blood cell stimulant used routinely by millions of patients with cancer around the world. Its use helped revolutionise bone marrow transplantation, and now stem cell transplantation, enabling cures to be offered to many more patients.
2. Venetoclax,<sup>10</sup> a novel anti-cancer targeted drug therapy, has replaced chemotherapy for chronic lymphocytic leukaemia and doubled survival for older patients with AML.
3. Molecular monitoring<sup>11</sup> of response to treatment of chronic myeloid leukaemia has transformed our use of targeted therapy, enabling personalised approaches, including cessation of therapy in many patients.
4. Pioneering of minimal residual disease detection and monitoring<sup>12</sup> to guide treatment of childhood acute leukaemia is now an essential ingredient in care to maximise the chance of cure, while reducing side effects from therapy.

Australia's comparative advantage in blood cancer research is in three main areas: blood cancer biology (including immunology), applied genetics research and clinical trials. All can be leveraged if there is sufficient investment through research funding that encourages long-term focus and collaboration.

## RESEARCH INVESTMENT MUST HAPPEN NOW

The need for blood cancer research is clear. Equally clear is the need to invest early, to frontload the research effort now to avert the predicted rise in blood cancer prevalence, incidence and mortality over the coming decade. Fundamental research is a critical element.



Image credit: Leukaemia Foundation

## **COMMONWEALTH FUNDING FOR BLOOD CANCER RESEARCH**

Presuming that the present triennium of funding remains constant at \$125 million, an additional \$125 million is needed (on the basis of blood cancer incidence doubling over the next decade), totalling \$250 million over the next triennium.

The Commonwealth Government must lead the charge; presently only 58% of all cancer funding is provided through Australian Government sources.<sup>13</sup>

Through the National Health and Medical Research Council (NHMRC) and the MRFF, the Commonwealth must be the major contributor of the \$125 million investment over and above existing funding for the triennium commencing 2025. The current pattern of government funding will not match the estimated doubling of blood cancer cases by 2035.

Existing MRFF-funded missions, such as the Genomics Health Futures Mission<sup>14</sup> and Stem Cell Therapies missions have only provided indirect benefits to blood cancer research.

A further increase in government funding beyond the next triennium will be required to curb the predicted growth in cases and deliver the actions outlined in the Research Roadmap below. There is an opportunity to continue to develop targeted funding programs to address the looming healthcare burden posed by blood cancers.

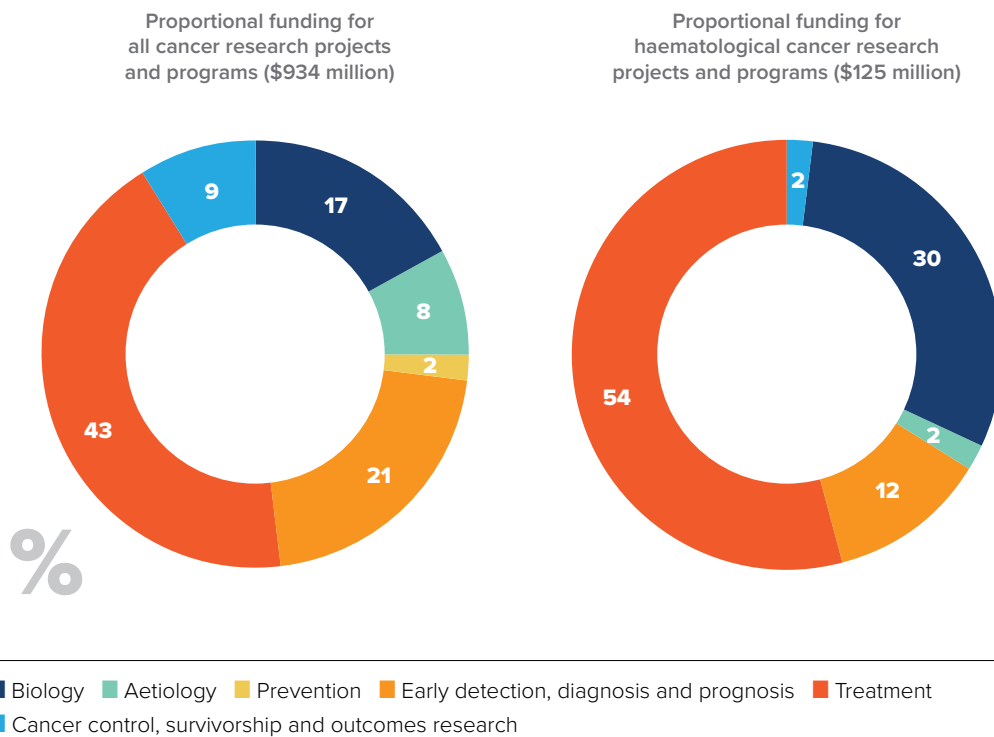
## RESEARCH INVESTMENT MUST BE REBALANCED

Current research funding for blood cancers needs to be enhanced and rebalanced. Across the entire research system (including infrastructure and workforce), attention is needed to the quantum, timeliness, distribution and underpinning enablers.

The distribution of funding for blood cancer research in Australia prior to the pandemic is different from the distribution of funding for cancer research overall. The difference is reflecting the fact that research priorities for blood cancers that are non-preventable with no national population screening programs are different from those for other cancers, such as breast, prostate, lung and bowel cancers.

Funding should therefore be directed to areas of greatest challenge, as well as areas where the current research trajectory suggests major advances are imminently possible with additional and early investment.

**Figure 1: Source: Cancer Australia – An overview of funding for cancer research projects and programs in Australia 2018–2020.**



## RESEARCH INVESTMENT MUST ENABLE COLLABORATION

Pivotal to research success is the need to encourage domestic collaboration. Australia's habit of atomising research funding into multiple packages and programs is not only inefficient, but also damaging. It forces research groups to compete for funding more than it encourages cooperation and appropriate collaboration at scale.

# A RESEARCH ROADMAP FOR SUCCESS

A national quest can focus and enhance research efforts towards cancer and healthcare issues at scale.

We propose a national quest to address the urgent and complex challenges posed by blood cancers in Australia: **zero preventable blood cancer deaths by 2035**.

This *Research Roadmap for Blood Cancer* maps a pathway to achieve zero preventable blood cancer deaths by 2035.

The Research Roadmap has been informed by the *National Strategic Action Plan for Blood Cancer*<sup>15</sup> and an earlier consultation paper. It has been designed to be focused, strategic and deliverable.

**The focus:** achieve the national quest of zero preventable deaths from blood cancer. More needs to be known about their complexity and their changing incidence.

**The strategy:** accelerate discovery now to avoid the negative outcomes of an increasing blood cancer caseload. Translate understanding into new treatments, care, clinical practice and future prevention.

**The delivery:** the Research Roadmap presents the practical steps to initiating and achieving the quest. It will be delivered by the blood cancer community, including researchers and research organisations, clinician-researchers, patients and advocacy groups, clinicians and allied health, industry, government, science agencies and philanthropy.

## HOW TO FOLLOW THE RESEARCH ROADMAP

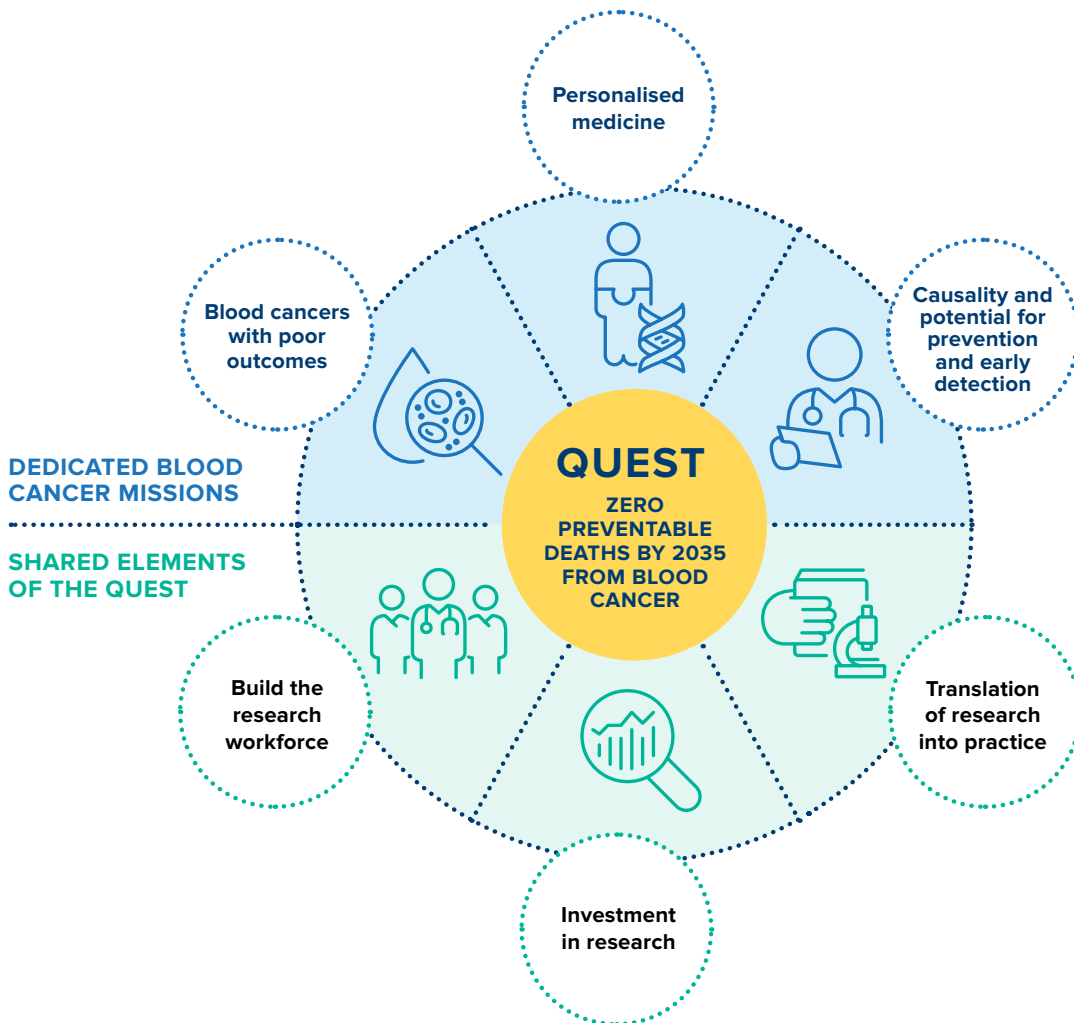
The Research Roadmap comprises:

- the overarching national quest
- three missions to address unmet needs
- three priority areas of focus with specific actions and timelines

The Research Roadmap should be accompanied by dedicated review points after one, three, five and seven years of implementation to ensure continual progress and relevance. These reviews should assess the suitability and impact of actions, allowing for necessary adjustments based on emerging scientific evidence, technological advancements, and evolving healthcare landscapes. By maintaining this flexibility, the Research Roadmap can adapt to new challenges and opportunities, ensuring sustained momentum toward the quest for zero preventable blood cancer deaths.

Continuous stakeholder engagement and transparent communication are important during implementation. Through an adaptive and iterative process, the Research Roadmap for Blood Cancer will drive innovation and collaboration and ensure that the quest for zero preventable deaths by 2035 remains a tangible and achievable goal.

## NATIONAL BLOOD CANCER QUEST



## BLOOD CANCER MISSIONS

Interwoven throughout the priority areas of the Research Roadmap are three missions to address currently unmet needs. At a minimum, the \$125 million investment into blood cancer research is required over and above the existing triennium. Funding needs to be spread across these three missions.



### **Mission 1: Blood cancers with poor outcomes**

The key to improving survival for blood cancers with poor prognosis (such as AML, poor prognosis multiple myeloma, recurrent aggressive lymphomas and currently intractable rare blood cancers) is the development of novel therapies, which can only be unlocked through dedicated fundamental research followed by translation into clinical practice.

This requires unravelling the complexities of blood cancers at a molecular level, paving the way for targeted therapies and personalised treatment strategies (Mission 3).

This mission is about making discoveries that create step change in how we approach these cancers. Translation of these research findings into clinical practice will be accelerated through programs and partnerships with pharmaceutical companies and healthcare providers, ensuring these discoveries meet the patients. The aims of each program should address the major barriers to progress.

We propose that this mission receive approximately 40% of new government funding for blood cancer research.



### **Mission 2: Causality and potential for prevention and early detection**

To get ahead of the rise in incidence, we need to know more. This mission aims to identify risk factors, genetic predispositions, and environmental influences contributing to blood cancers.

Funding interdisciplinary studies and a mix of 'blue-sky' research exploring the mechanisms underlying blood cancer development—such as genetic mutations, and immune system dysregulation—lays the groundwork for effective prevention strategies.

Translating these discoveries into prevention strategies will involve collaboration between public health agencies, healthcare providers and community organisations to disseminate findings and implement strategies, ultimately improving public health outcomes on domestic and global scales.

This mission should receive approximately 20% of new government funding for blood cancer research.





### Mission 3: Personalised medicine

This mission prioritises research investment into exploring biomarkers, genetic variations and molecular pathways specific to different types of blood cancer.

Selecting the right therapy for the right patient with the right diagnosis is a prerequisite to their care and their outcome. Traditional therapies may or may not be suitable to a given individual and their diagnosis, and for some, new therapies already in clinical trials may be the answer. Programs in this mission should focus on defining these parameters for blood cancers and their subtypes where standard therapies are failing a significant proportion of patients.

Through collaboration opportunities with pharmaceutical companies, regulatory bodies and healthcare providers, this mission aims to revolutionise blood cancer treatment paradigms and explore new clinical trials, registry studies, diagnostics and prognostics.

These programs should offer immediately implementable evidence that improves cancer burden to healthcare networks and improving patient and practice outcomes and quality of life.

We propose that this mission receive approximately 40% of a new government investment in blood cancer research.

## PRIORITY AREAS

To achieve the aim of zero preventable deaths from blood cancers by 2035, we need to leverage opportunities to **accelerate research advances** through:



**1. Investing in research:** Ensure funding for blood cancer research is sufficient in quantum and distributed for greatest impact.



**2. Translation of research into practice:** Improve the mechanisms for the latest evidence and technologies to be translated into practice.



**3. A skilled workforce:** Enhance the research workforce, including researchers and clinician-researchers, by investing in appropriate training, support and retention.



## Priority area 1: Investing in research

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### **a. Need for fundamental research into the full spectrum of blood cancers**

Fundamental or discovery research builds the foundational knowledge that underpins applications. It is driven by curiosity, rather than pursuing a specific application or commercial outcome.

The potential to unlock disease mechanisms, identify novel treatment targets, provide educational and training opportunities, improve diagnosis and prognosis, and improve long-term patient outcomes has to be realised given the predicted growth in blood cancer patients in Australia.

Without new knowledge, old treatments dominate; the opportunities for new, sometimes radically different treatments are fewer. Australian advancements in therapies such as Venetoclax, G-CSF and molecular monitoring during targeted therapy for chronic myeloid leukaemia were only made possible through fundamental discovery science.

The decline of investment in fundamental research in Australia must be reversed.

The potential to unlock disease mechanisms, identify novel treatment targets, provide educational and training opportunities, improve diagnosis and prognosis and improve long-term patient outcomes is so great, we must start now.

### **Early and enhanced investment in fundamental research is necessary to address the priorities of the quest and the blood cancer missions.**

Increases in the quantum of funding for fundamental research will reap the greatest rewards if it is managed in ways that promote two other essential characteristics: long-term investment and commitment to collaboration.

Complex problems also require a team of researchers or 'teams of teams' comprising investigators from multiple disciplines to make the big breakthroughs—funding must therefore encourage collaboration over competition.

The existing research funding system creates a counterproductive environment in which incentives encourage individuals to compete rather than collaborate.

Improving domestic collaboration and coordinating efforts across the blood cancer research community would be beneficial as a mechanism for building greater scale, and to use resources more effectively and efficiently, but would require the development of an incentive scheme.

Australia currently lacks substantial funding to support researchers to join large international consortia or joint research programs. Joining large international projects can bring substantial benefits: expanding available patient cohorts, collaborating with world leading experts, and gaining access to infrastructure. Schemes should be established to enable Australian researchers to participate in international research collaborations.

## FUNDAMENTAL RESEARCH INTO THE FULL SPECTRUM OF BLOOD CANCERS

**What are the biggest problems?** The causes of the growth in blood cancer prevalence in the Australian population are not known, and there are no effective therapies for blood cancers with low survival rates.

**What can we do to address these?** Fund research at the appropriate level and duration because it is fundamental to minimising the worst outcomes for Australians—and to achieving zero preventable deaths by 2035.

Action	Year actioned
Fund research into the biological bases of blood cancers and their complexity, with emphasis on poor prognosis blood cancers (Mission 1).	2025
Fund research into why the prevalence is increasing in Australia (Mission 2).	2025
Develop performance incentives that reflect and encourage collaboration between blood cancer researchers from different disciplines (Missions 1, 2 & 3).	2025
Create schemes to enable Australian researchers to participate in international research collaborations (Missions 1, 2 & 3).	2025

**What success looks like:** A robust research pipeline grounded in strong fundamental research and driven by collaborative, multidisciplinary teams.

**Key metrics:** Increased multidisciplinary research collaboration and publications. Major Australian discoveries each year, including through Australian contributions to international collaborative research. Increased international investment into Australian blood cancer research.

### b. Need to enhance prevention, early detection and diagnostics

Early detection and diagnosis have improved outcomes in solid tumour cancers such as breast and colorectal cancers. However, their impact on blood cancers has been minimal to date.

Australia needs to concentrate investment on exploring whether blood cancers can be prevented, detected or diagnosed early. Strategies, including genomic screening, could lead to opportunities for therapeutic intervention at an earlier stage than is currently possible. This could lead to a decrease in incidence and improved outcomes. While we know that diagnosis at an earlier stage is associated with a better prognosis in some blood cancers, e.g. Stage I/II B-cell lymphomas, historically for most blood cancers earlier diagnosis or treatment has not been associated with improved outcomes.

Therefore, investment in early detection should be directed towards situations where outcomes can be improved. For example, certain patients who have a predisposition for blood cancers, or are exposed to known leukaemia-inducing agents. These represent identifiable subgroups where research-based early detection and prevention strategies should be developed.

The *Australian Cancer Plan*<sup>16</sup> should be expanded to incorporate evidence-led opportunities and implement risk stratification models. These models would help identify individuals at higher risk of developing blood cancers.

Advancements in imaging and biomarkers could enable the early detection and characterisation of different blood cancers. This would require investment in research that leverages patient registries and in technology.

## ENHANCE PREVENTION, EARLY DETECTION AND DIAGNOSIS

**What's the biggest problem?** For patients with a high risk of developing poor-prognosis blood cancers.

**What can we do to address this?** Focus research into whether early detection and prevention strategies may benefit people at high risk of developing fatal blood cancers.

Action	Year actioned
Fund research into early detection, diagnosis and potential prevention—using the same principles as outlined for fundamental research (Mission 2).	2025
Expand the <i>Australian Cancer Plan</i> to incorporate and implement risk stratification models based on demographic, clinical and genetic factors that allow identification of individuals at higher risk of developing blood cancers (Missions 1, 2 & 3).	2027

**What success looks like:** Earlier detection and diagnosis for highest risk patients. Availability of timely interventions that are prospectively assessed in research-led care.

**Key metric:** Reduced number of preventable deaths from blood cancers with a known predisposition.

### c. Build informative and accessible research collections and biobanks

Large-scale shared research collections—particularly registries and biobanks—are the international gold standard. These biobanks collect vast amounts of data, which are used to build and test hypotheses as knowledge develops.

Australia has several registries focusing on different types of blood cancers, such as AML,<sup>17</sup> lymphoma,<sup>18</sup> myeloma<sup>19</sup> and paediatric leukemia.<sup>20</sup> Notwithstanding, there is currently no national biobank or similar research infrastructure. Clinical data and biological samples are essential for blood cancer research.

Biobanks provide both domestic and international opportunities for sharing and analysis of specific datasets on various demographics and populations. International examples, like the UK Biobank,<sup>21</sup> have shown how nationwide comprehensive data and biospecimen collection is a valuable resource for investigating epidemiology, disease incidence, prevalence, treatment patterns and outcomes.

The ability to leverage all available datasets in Australia to answer key research questions is particularly relevant for the rare and very rare subtypes of blood cancers. This can aid in developing personalised treatments.

Currently, Australian research datasets are not unified or standardised and can be difficult to find and access. Some have potentially outdated frameworks or standards and time-consuming barriers such as negotiating access and differential systems, policies, processes and formats.

Seamless access to these research collections has the potential to maximise collaboration and increase time spent on actual research rather than data reformat and linkages. Aggregated datasets enable trend analysis, including identifying disparities in care or diagnosis. They can inform clinical practice and health policy decisions, disease mechanisms, prevention strategies, and high-risk populations for targeted screening and intervention programs.

Investing in a national cancer registry with a specific branch dedicated to blood cancers would serve as an invaluable resource to increase collaboration, and improve our understanding of disease, diagnosis, treatments and follow-up through cancer survivorship.

BUILD INFORMATIVE AND ACCESSIBLE RESEARCH COLLECTIONS AND BIOBANKS	
<p><b>What's the biggest problem?</b> Access to data and biospecimens is inadequate, patchy, and insufficiently coordinated and resourced in Australia.</p> <p><b>What can we do to address this?</b> Invest in both general biomedical research infrastructure funding and in specific facets that are central to effective blood cancer research.</p>	
Action	Year actioned
Ensure blood cancer data and biospecimens are specifically catered for in the implementation of the <i>Australian Cancer Plan</i> (Missions 1, 2 & 3).	2025
Incentivise and invest to allow existing data sources and biospecimen repositories to harmonise using internationally accepted standards (Missions 1, 2 & 3).	2025
Develop data platforms tailored for blood cancer research including genomic, clinical, lifestyle and environmental data (Missions 1, 2 & 3).	2030
<p><b>What success looks like:</b> Interoperable and accessible blood cancer health data and biospecimens, capturing Australia's uniquely diverse population and challenges.</p> <p><b>Key metrics:</b> Integration of health data with biospecimen research data. Research collections and biobanks are representative of the whole Australian population. Usage metrics for collections are captured, including number of academic collaborations fostered, research programs supported, publications and discoveries.</p>	



## Priority area 2: Translation of research into practice

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### a. Translation transforms outcomes

Research is delivering transformative improvements in outcomes for some patients with blood cancer. For instance, some blood cancers that used to have poor prognoses now have greatly improved treatment outcomes: acute promyelocytic leukaemia now has high survival rates, survival for childhood acute lymphoblastic leukaemia has risen from zero to over 90% over the past several decades and chronic myeloid leukaemia has near-normal life expectancy (see Appendix).

Another recent example is the ZERO childhood cancer program,<sup>22</sup> which allows every child in Australia to have access to precision medicine. This program provides personalised medical treatment, transforming the diagnosis of childhood cancers by pinpointing their molecular basis and improving clinical outcomes and survival.<sup>23</sup> The opportunity of this program's success in improving long-term survivorship outcomes—such as organ damage, fertility and predisposition to secondary cancers—underscores the potential of personalised medicine if it can be broadened for all types of cancer across a broader demographic.

Translating breakthroughs in fundamental understanding of blood cancer should be able to improve outcomes for patients; but it requires concerted investment and collaborative efforts. In recent years significant investments have been directed at translating research into commercial products.<sup>24</sup>

Australia needs a policy framework that incentivises translation of research into products and services. Promoting knowledge exchange platforms for sharing research findings, best practices, and success stories from blood cancer research across government, academia and industry would assist in accelerating innovation, fostering collaboration, avoiding duplication of efforts and encouraging the rapid translation of research into effective treatments and policies.

Dedicated funding streams under the MRFF would help by providing targeted resources for high-priority areas in blood cancer research, enabling the translation of research findings into treatments and interventions. These areas should include hard-to-target and hard-to-treat blood cancers, and high-mortality blood cancers.

Focusing research translation funding opportunities on areas of blood cancer research where Australia has a comparative advantage will be beneficial, as it will leverage Australia's strengths in expertise and infrastructure.

## TRANSLATION OF AUSTRALIAN DISCOVERIES

**What's the biggest problem?** Delays in translation of research outcomes into Australian clinical care.

**What can we do to address this?** Apply research breakthroughs and advances to tackling blood cancers where patients have poor outcomes. Develop a policy framework with aligned funding and incentives to translate research into improved patient outcomes.

Action	Year actioned
Dedicated MRFF funding streams that translate research into tackling hard-to-treat and high-mortality blood cancers (Mission 1).	2025
Focus research translation funding opportunities to areas of Australian comparative advantage (Mission 3).	2025
A policy framework to encourage Australian entities to translate research into products and services that enhance patient care (Missions 1, 2 & 3).	2025
Promote knowledge exchange platforms for sharing research findings, best practices and success stories across government, academia and industry (Missions 1, 2 & 3).	2027

**What success looks like:** Increased translation of research into products, services and policies by Australian entities.

**Key metric:** Growth in Australian patents related to blood cancer (medium-term), and ultimately increased access to new diagnostics and treatments for Australian patients (longer-term).

### b. Advance personalised medicine

Precision diagnostics, predictive biomarkers, tailored treatment approaches and targeted therapies can optimise healthcare through more efficient resource allocation and expenditure. These approaches are particularly beneficial to blood cancers given the complexity and diversity of subtypes, each presenting very different and unique challenges for treatment and benefits from targeted approaches.

Successful programs like the ZERO program run by the Children's Cancer Institute in partnership with the Kids Cancer Centre at Sydney Children's Hospital Randwick, and the MoST-LLy program through QIMR Berghofer<sup>25</sup> are becoming more readily available for blood cancer patients (including the adapted MoST-LLy program for advanced or poor-prognosis blood cancers). These programs offer the opportunity to test and analyse a cancer's unique genetic profile. This provides the referring practitioner data detailing genetic biomarkers and suitable personalised treatment options.

The exploration of mRNA technology for blood cancers is gaining momentum, presenting an opportunity to build on recent government and industry investments. mRNA-based treatments can be customised to target specific proteins, potentially enhancing outcomes in some harder-to-target blood cancer types.

Australia should invest in further research in personalised medicine, including genomics and mRNA technologies. Integrating patient-specific genetic information, disease characteristics and treatment responses is crucial to developing precision therapies that minimise toxicities and enhance overall survivorship in blood cancer patients.

This approach is further enhanced by studying biomarkers (genetic, molecular, or imaging based) that can predict individual responses to treatments and guide personalised therapeutic interventions.

Onshore data centres and secure computing infrastructure within Australia are critical for managing, analysing and protecting large volumes of sensitive data, while also facilitating research and ensuring data sovereignty and patient privacy. These data and associated infrastructure enable personalised medicine and omics methods in paediatric and adult populations.

ADVANCE PERSONALISED MEDICINE	
<p><b>What's the biggest problem?</b> One-size-fits-all cancer treatments are not the best therapy for blood cancers.</p> <p><b>What can we do to address this?</b> Develop targeted therapies through increased investment and research into innovative technologies, such as targeted and cellular therapies, genomics and mRNA.</p>	
Action	Year actioned
<p>Australia should develop and invest in capacity for personalised therapies (Mission 3). Specifically:</p> <ul style="list-style-type: none"> <li>• genomic sequencing routine diagnostics to underpin integration of personalised medicine in practice</li> <li>• targeted therapies and cellular therapies</li> <li>• mRNA technologies and other omics technologies for diagnostics and therapeutics that are fit for purpose for blood cancers with poor outcomes.</li> </ul>	2025
<p>Australia needs to invest in research programs to identify biomarkers (genetic, molecular, or imaging based) that can predict individual responses to treatments and guide personalised therapeutic interventions (Missions 1, 2 &amp; 3).</p>	2027
<p><b>What success looks like:</b> Patients get the right diagnosis and the optimal initial treatment. A reduced disease burden and improved patient outlook and survivorship experience for patients.</p> <p><b>Key metric:</b> Increased initial diagnosis accuracy. Increased personalised treatment options for Australian patients, especially patients with blood cancers with a poor prognosis.</p>	



### c. Embed clinical trials

Australia maintains a good reputation for quality research and clinical trial expertise, making it an attractive destination for clinical trials. The Therapeutic Goods Administration also has an advantage in flexibility and speed over regulators in other regions, such as Europe.<sup>26</sup>

Navigating our clinical trial system can be complex and confusing for patients and researchers, which potentially reduces the number of trial participants. Although our research reputation and clinical trial outputs are attractive, our small population size and geographical isolation make it challenging to attract major pharmaceutical and biotech industry support.

A national and consistent approach to clinical trials, including implementation and regulation, is needed to deliver improved health outcomes and innovative treatments.

An Inter-Governmental Policy Reform Group is developing a national system for clinical trials that benefits patients, researchers, and healthcare workers.<sup>27</sup> This will involve streamlined regulatory processes and efficient ethics approval processes that will expedite the initiation of clinical trials, allowing rapid evaluation of novel treatments and interventions.

As part of this reform, clinical trials of new treatment options specific to blood cancers should be targeted for development. A national target of 80% of in need patients such as those with poor prognosis or poor response to standard therapies could then be referred to relevant clinical trials to increase access to cutting-edge care.

This could accelerate drug development, enhance the patient experience, enable more precise patient stratification, foster increased collaboration and data sharing and improve regulatory efficiency and flexibility.

#### INCREASE SUPPORT FOR PROSPECTIVE DATA COLLECTION AND CLINICAL TRIALS

**What's the biggest problem?** Clinical trials in Australia are difficult to navigate for clinicians, researchers, patients and primary carers.

**What can we do to address this?** Reform Australia's clinical trial framework to be effective, efficient, and maintain a focus on the patient.

Action	Year actioned
Benchmark percentage of patients in each disease and jurisdiction that are entered into registries and clinical trials (Missions 1, 2 & 3).	2027
Establish a long-term, secure and tailored funding stream to support clinical trials, including support for innovative clinical trial concepts (Missions 1, 2 & 3).	2025
Implement a long-term, secure and performance-based funding stream to support clinical registries of highest national priority (Missions 1, 2 & 3).	2025
Review success of enrolment in clinical trials in particular blood cancer patient cohort (Missions 1, 2 & 3).	2027

**What success looks like:** Successful application of experimental treatments and diagnostics are improving Australia's cancer burden and blood cancer patient outcomes.

**Key metric:** Increased access and participation in clinical trials. Improved quality and quantity of data about specific blood cancers with high unmet need. Improved linkage between clinical registries and clinical trials.

#### d. Expanding health systems research

Wherever Australians live, and no matter their social or cultural background, they have a right to expect access to quality treatment and care.

Existing patterns of care show a disproportionate impact on rural and remote communities and populations. Rural and remote patients are disadvantaged by: geographical isolation; lack of access to advanced diagnostics, treatments and care; and, often, higher out-of-pocket expenses. These disparities in access increase risks associated with delayed diagnosis, limited access to specialised care and even fewer treatment options.

It is important to know how patients can most effectively interact with the system to maximise their benefits.

Establishing how patients can best be supported—including psychologically and socially—will support both routine care and enable breakthroughs in clinical and biomedical research on blood cancers.

ENABLING EQUITABLE ACCESS TO HEALTHCARE	
<b>What's the biggest problem?</b> Inequitable access to healthcare.	
<b>What can we do to address this?</b> Commission health systems research to identify limitations that can be corrected and generate solutions such as telemedicine and telehealth.	
Action	Year actioned
Invest in health systems research (Missions 2 & 3). Specifically: <ul style="list-style-type: none"><li>• identify inefficient care and gaps in delivery of high-value care</li><li>• test proposed solutions to resolving weaknesses</li><li>• identify and implement the most effective ways to educate and inform patients at the key stages of diagnosis, treatment and during survivorship</li><li>• study patient involvement in decision-making processes related to their treatment plans and options</li><li>• identify how Australians in remote or under-served regions can readily access comprehensive diagnosis, treatment, supportive care and research tailored specifically for blood cancers.</li></ul>	2025
<b>What success looks like:</b> Evidence-informed and delivered patient-centric care to Australia's widely distributed population.	
<b>Key metrics:</b> Higher adherence to Optimal Care Pathways. Reduced time to access definitive diagnosis. Better access to treatment for those in rural and remote communities.	



## Priority area 3: Skilled workforce

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### a. Build the researcher workforce across all disciplines and career stages

The quest to achieve zero preventable deaths from blood cancers by 2035 necessitates a diverse and future-proofed research workforce. The development of such a research workforce hinges on our ability to attract, train, retain and mentor researchers and experts in cell and molecular biology, epidemiology, drug discovery, biobanking, policy development, haematology, oncology and pathology.

Given the complexity and diversity of blood cancers, both specialised fundamental knowledge and interdisciplinary approaches are required. Strong fundamental research disciplines are essential as they provide a knowledge base that can be drawn upon for future applications and for multidisciplinary collaborations.

Researchers need to be trained with the ability to adapt to new technologies and methods, equipped with the fundamentals of scientific inquiry and encouraged to collaborate and share knowledge both domestically and internationally. If certain skills are not available domestically, support should be provided for exchange programs.

Building and growing the necessary research workforce will require a national strategy and implementation plan. Many researchers leave the field early in their career due to instability of employment, reliance on grant funding, and the difficulty of obtaining fellowships. Providing support for researchers with multidisciplinary expertise, including medical science technology, epidemiology, drug development and clinical trials at the early stages of their career could result in higher retention of blood cancer researchers.

#### Clinician researchers

Clinician-researchers—who play an intermediary role between clinicians and discovery science—are instrumental in translating research into the healthcare system. They ensure Australians get the best care possible and use their clinical experiences to inspire their research.

The Australian Academy of Health and Medical Sciences conducted a detailed analysis of the challenges faced by this group in their 2022 report *Research and innovation as core functions in transforming the health system: A vision for the future of health in Australia*.<sup>28</sup> The report highlighted challenges, including limited funding for research activity, insufficient time allocated for clinician-researchers to undertake research, and a lack of support from some senior healthcare executives. Overall, clinician-researchers experience slower career progression compared to their colleagues. Under the current system, they often need to secure two positions—one clinical and one research—to facilitate their work, usually liaising with two different employers.<sup>29</sup>

#### Broad, multidisciplinary research workforce

Where it exists, research training is aimed at specialist medical doctors and often requires the sacrifice of personal financial stability in a highly competitive research area.

There is an opportunity to provide formalised training programs and specific research training within one or more areas to expand and diversify this workforce to include nurses, allied health professionals, pharmacists, and clinical trial practitioners.<sup>30</sup>

Strategic approaches to developing formal clinician-researcher models have been successful internationally—for instance, in the United Kingdom and Canada.<sup>31</sup> Although Australia's health system differs, these models provide a framework that can be adapted to our local context and applied in metropolitan, rural and remote settings.

## PLAN TO GROW AND RETAIN THE RESEARCH WORKFORCE

**What's the biggest problem?** The research workforce is inadequate for the blood cancer problem.

**What can we do to address this?** Develop and support a workforce with the requisite skills.

Action	Year actioned
A national strategy and implementation plan for building the world-class scientific research workforce needed to drive the necessary fundamental, translational, clinical and health services research in blood cancers (Missions 1, 2 & 3).	2027
Develop a harmonised clinician-researcher training and career pathway, such as a clinical fellowship training scheme to train a future secure workforce with multidisciplinary expertise (Missions 1, 2 & 3).	2026
Provide support, mentorship programs and educational initiatives for early- and mid-career researchers to develop their skills, and ultimately to retain and grow the blood cancer researcher workforce (Missions 1, 2 & 3).	2027
Provide research training and joint clinician-researcher pathways to healthcare workers and medical scientists and technologists, including nurses, allied health professionals, epidemiologists, pharmaceutical scientists and pharmacists, to expand and diversify the clinician-researcher workforce (Missions 1, 2 & 3).	2025

**What success looks like:** Improved performance, capacity, reputation and longevity of our domestic blood cancer research workforce. Attraction of top researchers and research groups to Australia.

**Key metric:** Increased numbers and retention of blood cancer researchers in Australia across each career stage and research discipline.

# APPENDIX

The incidence of blood cancer will increase significantly between 2022 and 2035, based on projections completed by Insight Economic in the *State of the nation: Blood cancers in Australia 2023* report and Australian Institute of Health and Welfare data.<sup>32</sup>

More than 108,000 adults aged 25 to 65 will be diagnosed with blood cancer. The leading subtype will be non-Hodgkin lymphoma. More than 7,000 children and 6,000 adolescents (ages 15–25) will be diagnosed with blood cancer, with the leading types of acute lymphoblastic leukaemia, acute myeloid leukaemia, non-Hodgkin lymphoma and Hodgkin lymphoma.

The 65+ age bracket is expected to see more than 250,000 people diagnosed with blood cancer, with leading diagnoses including myeloma, chronic lymphocytic leukaemia, myelodysplastic syndromes and non-Hodgkin lymphoma.

For the period 2015–2019, the five-year observed survival rate for all Australians with blood cancers was 60.7%, increasing by 2.2% since the last four-year period (2010–2014).

The blood cancers with the least favourable observed five-year prognosis from 2015 to 2019 are summarised in Table 1, and for comparison, those observed from 1990 to 1994 are summarised in Table 2.

**Table 1: Blood cancer subtypes with the least favourable prognoses, 2015–2019**

Survival type	Cancer group/site	Period (years)	Sex	Years after diagnosis	Survival (%)
Observed	Other and unspecified leukaemia	2015–2019	Persons	5	19.0%
Observed	Acute myeloid leukaemia	2015–2019	Persons	5	24.9%
Observed	Other and unspecified myeloid leukaemia	2015–2019	Persons	5	29.1%
Observed	Myelodysplastic syndromes	2015–2019	Persons	5	30.1%
Observed	Chronic myelomonocytic leukaemia (including juvenile)	2015–2019	Persons	5	32.1%
Observed	Multiple myeloma	2015–2019	Persons	5	50.5%
Observed	Leukaemia	2015–2019	Persons	5	57.7%
Observed	All blood cancers combined	2015–2019	Persons	5	60.7%
Observed	Non-Hodgkin lymphoma	2015–2019	Persons	5	68.0%
Observed	Myeloproliferative neoplasms (excluding CML)	2015–2019	Persons	5	68.1%
Observed	Lymphoma	2015–2019	Persons	5	70.1%
Observed	Myeloproliferative neoplasms	2015–2019	Persons	5	70.2%
Observed	Other and unspecified lymphoid leukaemia	2015–2019	Persons	5	71.8%
Observed	Acute lymphoblastic leukaemia	2015–2019	Persons	5	72.7%
Observed	Chronic lymphocytic leukaemia	2015–2019	Persons	5	74.2%
Observed	Other blood cancers	2015–2019	Persons	5	76.2%
Observed	Chronic myeloid leukaemia (CML)	2015–2019	Persons	5	77.3%
Observed	Hodgkin lymphoma	2015–2019	Persons	5	85.8%

**Table 2: Blood cancer subtypes with the least favourable observed prognoses, comparison 1990–1994 and 2015–2019**

Blood Cancer Type	1990–1994 five-year survival	2015–2019 five-year survival
Acute Myeloid Leukaemia	10.9%	24.9%
Multiple Myeloma	23.0%	50.5%
Non-Hodgkin Lymphoma	44.0%	68.0%
Acute Lymphoblastic Leukaemia	54.9%	72.7%
Chronic Myeloid Leukaemia	28.2%	74.2%
Chronic Lymphocytic Leukaemia	59.9%	74.2%
Hodgkin Lymphoma	76.0%	85.8%

Major advances in diagnosis, prognosis and survival are only made possible through research. The improvements in outcomes can be dramatic. Chronic Myeloid Leukaemia is a perfect example. In 1990–94, five-year survival rates were 28.2%. The advent of transformative new targeted therapies and molecular diagnostics delivered an increase in five-year survival rates to 77.3%. Even those astonishing population statistics understate the benefits from research into this disease. Indeed, the majority of those patients diagnosed with CML in the 2020s have a near normal life expectancy.

Clearly, the key to tackling the current unacceptably low rates of five-year survival is the research highlighted in this Research Roadmap. What these grouped statistics do not show is that some subtypes of leukaemia, lymphoma and myeloma have very poor prognosis. This is the challenge in 2025. Major gains are needed for poor prognosis blood cancers and that is the focus of Mission 1.

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