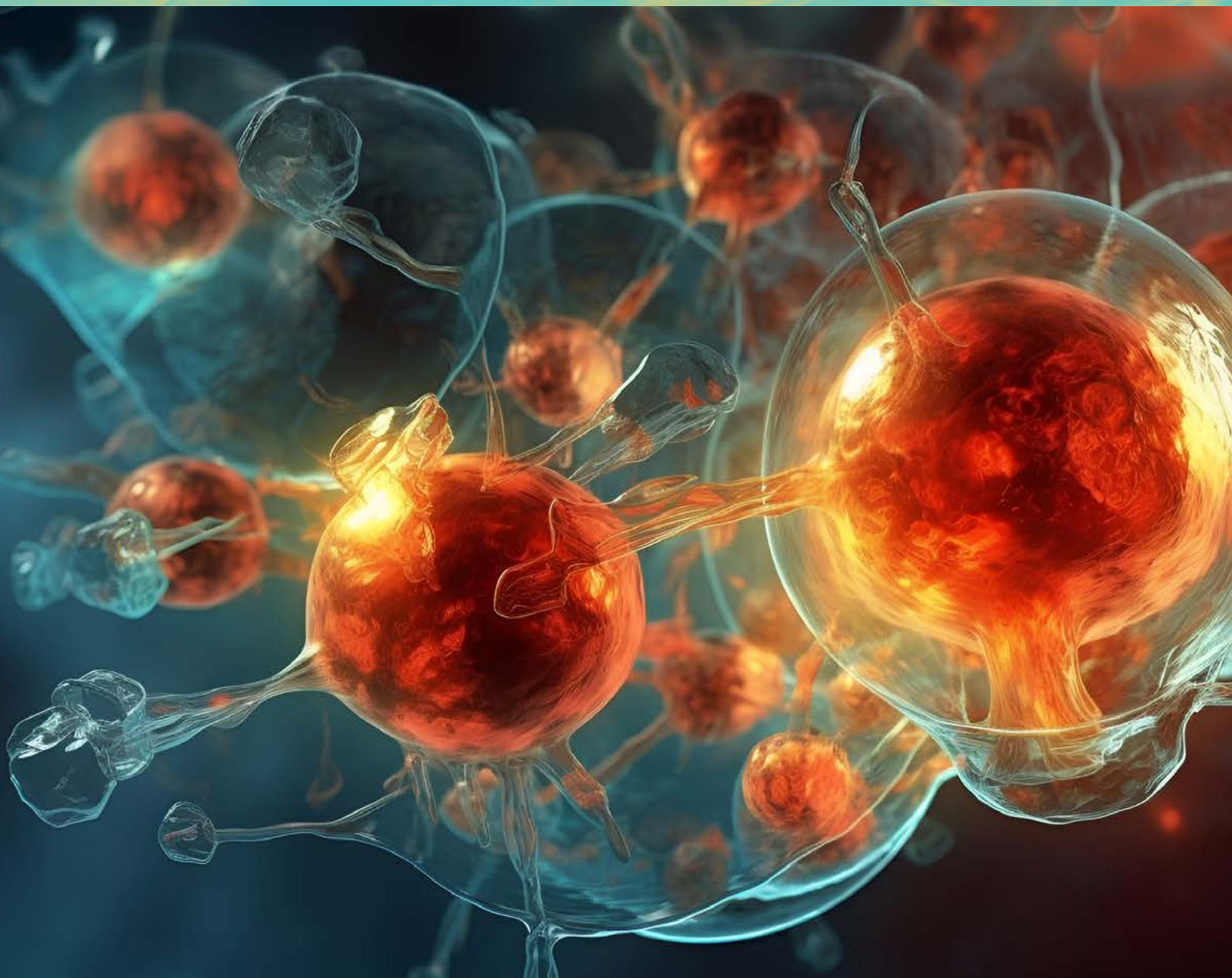
Australian Academy of Science

Australian Academy *of* Health and Medical Sciences

RESEARCH ROADMAP FOR BLOOD CANCERS
CONSULTATION PAPER

NOVEMBER 2023



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ACKNOWLEDGEMENTS

Leukaemia Foundation®

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We would like the opportunity to thank our Expert Steering Group for their project guidance and oversight.

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CONTENTS

GLOSSARY

Aetiology: the reason(s) or cause(s) for a condition.

Apoptotic/apoptosis: cell pathway that induces cell death.

Applied research: research that seeks to address or solve a problem.

Artificial intelligence (AI): computerised systems which can perform tasks usually requiring human intelligence, such as visual perception, learning and problem solving.

Blood cancer: blood cancers encompass a range of malignancies that develop in the bone marrow, and impact the production and function of blood cells. While blood cancers are generally referred to as one of three types—leukemia, lymphoma, or myeloma—there are over 100 unique blood cancer subtypes identified to date.

Blood disorder: a condition that impacts the function or production of blood cells.

Cancer: a condition that causes uncontrolled cell growth.

CAR T-cell therapy: a type of immunotherapy that extracts samples of immune cells from the patient and genetically modifies them in a laboratory to recognise, seek out and kill cancer cells, before putting them back into the patient's blood system to fight the cancer.

Cytotoxic: something that is toxic to living cells.

Digitalisation: the adaptation of systems to be stored, managed and/or utilised digitally via computers and the internet.

Epigenetic dysregulation: abnormal or impaired regulation of gene expression not involving base-pair (nucleotide) changes in the DNA.

Epigenetics: chemical modifications to genetic material that modify gene expression.

Fundamental (basic) research: research that seeks to advance theoretical knowledge.

Germline genetic testing: analysis of genomic variations that a person is born with.

Hypoxia: when there is not enough or a below-normal level of oxygen.

Ischaemic heart disease: a condition where there is reduced blood supply to the heart, resulting in hypoxia.

Immunotherapies: therapies that harness an individuals' immune system to target/treat a condition.

Leukaemia: a type of blood cancer that occurs in the white blood cells.

Lymphoma: a type of blood cancer that occurs in the lymphatic system.

Machine learning: a type of artificial intelligence that harnesses algorithms to simulate human-like learning, including classifying data, making predictions, and identifying patterns.

Malignancy: a condition resulting from uncontrolled cell growth.

Microbiota: microorganisms (particularly in the gut) that can stimulate the immune system, break down threats in food compounds, and synthesise vitamins, enzymes, and amino acids.

Monoclonal antibodies: are designed to bind to cancer cells and expose them to the patient's immune system, or to guide targeted treatments to the cancer cell.

Mutational burden: the number of mutations (genetic changes) present in a cell.

Myeloma: a type of blood cancer that occurs in the plasma cells.

Not-for-profit: a not-for-profit organisation operates for a collective, public or social purpose, as opposed to for profit or personal gain.

Philanthropic: describes an individual or organisation seeking to impact positive change. Philanthropic donations provide alternative funding for research activities.

Precision medicine: medicine that uses molecular or genetic profiling to more efficiently select treatment and optimise healthcare outcomes.

Primary cancer: denotes the cell or tissue type where the cancer first developed (i.e. where the cancer starts).

Pro-apoptotic agents: agents that directly target apoptotic pathways to induce cell death.

Remission: when the symptoms of a disease decrease or disappear.

Secondary cancer: a cancer that originated from another cell or tissue type and/or from another place in the body.

Somatic genetic testing: an analysis of genetic variation in tumours to identify changes that can contribute to cancer.

Targeted therapy: a type of drug treatment, attacking specific cancer cell features (molecular targets) to stop the cancer growing and metastasizing. This is sometimes called molecular therapy or biological therapy.

Tumour microenvironment: the environment surrounding tumour cells including blood vessels, extracellular matrix, immune cells, fibroblasts, and cell signalling molecules.

Universal healthcare: a healthcare system in which all individuals have access to essential healthcare when and where they need it, without financial burden.

1. PURPOSE

The Australian Leukaemia Foundation has engaged the Australian Academy of Science (the Academy) to conduct scoping work for a 10-year Research Roadmap that accelerates breakthrough blood cancer research in Australia.

1.1 SCOPE

This consultation paper seeks to examine the current blood cancer research landscape in terms of clinicians, researchers, and patients. It aims to gather input on potential barriers, strengths, and opportunities that could be addressed and leveraged to improve public health outcomes and advance key areas of fundamental research.

This paper is designed to elicit feedback only and does not represent the Academy's position or views. The consultation period will be open from 10 November 2023 for a period of one month.

The webinars will be conducted in November 2023. Pre-surveys required for the webinars will be sent to registered attendees before the webinars are conducted.

The following are out of scope for the consultation and Research Roadmap process:

- Pharmaceutical Benefits Scheme medicines and regulation of pharmaceuticals
- Centrelink and social support funding
- Medicare and health insurance, including bulk billing practices
- Blood cancer models of care and clinical guidelines
- Recommendations regarding the allocation of specific research funding.

1.2 THE PROCESS

WEBINAR 1: CURRENT BLOOD CANCER RESEARCH LANDSCAPE
To identify pivotal areas warranting concentrated research efforts through dynamic discussions and data-driven interactions.
Date: Tuesday 21 November 2023 **Time** 3.00 – 4.30pm

WEBINAR 2: TECHNOLOGICAL ADVANCEMENT IMPACTS ON BLOOD CANCER RESEARCH
Focus on recognising avenues where Australia can assert its research sovereignty. This will pinpoint innovative approaches and opportunities that position Australia at the forefront, showcasing its unique contributions to the global blood cancer research landscape.
Date: Wednesday 22 November 2023 **Time:** 3.00 – 4.30pm

WEBINAR 3: THE WAY FORWARD
Actionable approaches to advancing research in priority areas and encouraging clinical translation of new innovations in a measurable and scalable way. This will identify tangible measures and strategic frameworks to accelerate progress in blood cancer research and clinical care in Australia.
Date: Thursday 23 November 2023 **Time:** 3.00 – 4.30pm

2. INTRODUCTION

When subtypes are considered together, blood cancers[1] are among the most common cancers (second highest incidence) and are the biggest contributors to cancer-related death in Australia (responsible for almost 6,000 cancer-related deaths annually).

The healthcare system in Australia can be complex. For a serious diagnosis such as blood cancer, a patient often has difficulty navigating[2] across multiple health professionals as well as between public and private health insurance scope of care.

The optimal care pathways[3] give an integrated model of cancer care that puts the patient's needs first, along with the best of technical care, regardless of ones' financial or insurance situation. These plans provide a national standard, aiming to ensure high-quality cancer care reaches across all of Australia.

The pathways include involvement from all health professionals including doctors (i.e. surgeons, oncologists, haematologists, radiologists, general practitioners, and others), and allied health professionals (i.e. pharmacists, nurses, managers of cancer services, and others).

If we are to see improvement in clinical care and survival of blood cancers, new priorities and significant progress across research, new therapies, and care must be made and implemented into the national standard of care.

The National Strategic Action Plan for Blood Cancer[4] sets out four key goals:

1. Empower patients and their families
2. Achieve best practice
3. Accelerate research
4. Enable access to novel and specialised therapies

The Research Roadmap will encompass various domains such as blood cancer biology, diagnosis, genomics, microbiota, epidemiology, diagnostics, immunotherapies, targeted or cellular therapies, and a comprehensive framework for monitoring implementation.

The implementation of this roadmap will be conducted by the blood cancer community within Australia and will be led by the Leukaemia Foundation, the Blood Cancer Taskforce, and state and federal governments.

2.1 HOW FAR HAVE WE COME?

Modern advances in research and technology have increased survival rates for blood cancers—particularly through early detection, and characterisation of specific cancer subtypes that has facilitated more personalised treatments.

Our increased understanding of blood cancer subtypes calls for a change to traditional research approaches. It is imperative that strategic research collaborations are implemented to accelerate the development of individualised treatment and management of blood cancer patients with emphasis on immunotherapies, and targeted and curative therapies.

The field of medicine is under constant strain and is always changing. These challenges offer opportunities for researchers and clinicians to adapt and innovate, especially with digital technology A tangible example of such adaptation and innovation is the use of telehealth appointments to manage patients with blood cancers.

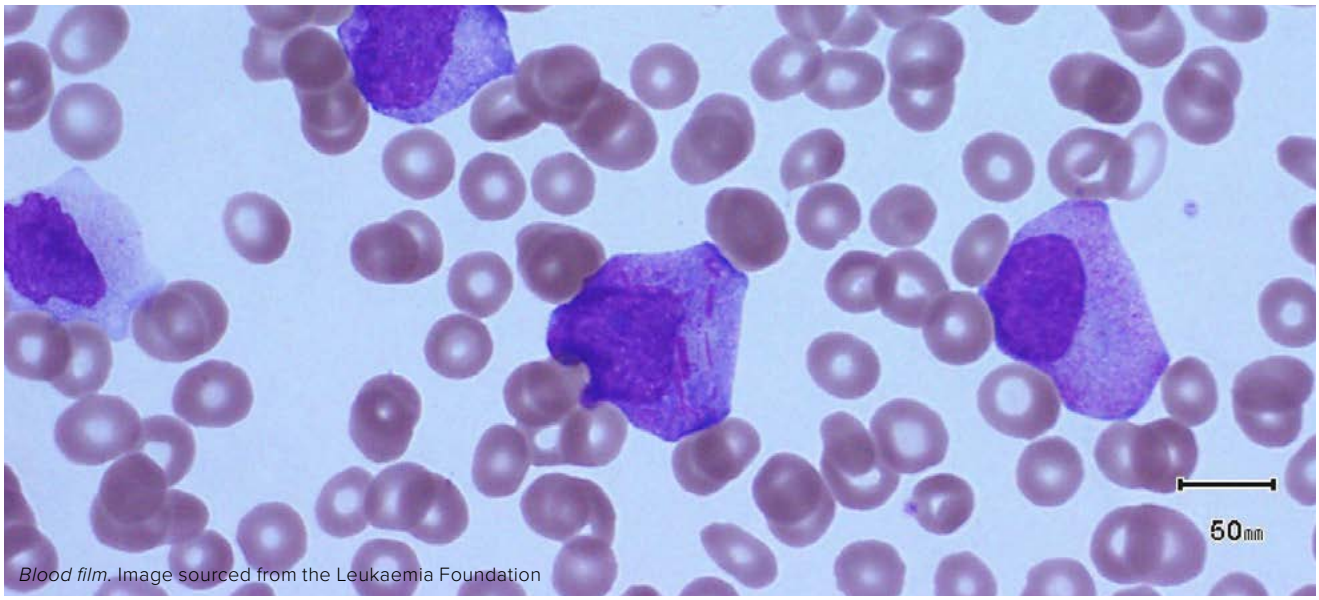
During the recent SARS-CoV-2 pandemic, telehealth services[5] were utilised across 18 million patients between 13 March 2020 and 31 July 2022. More than 95,000 practitioners use or have used the service, exceeding 118 million telehealth appointments of which, a subset may be blood cancer patients.

Although initially used to prevent the spread of the pandemic, these telehealth appointments serve as an example of rapid change and became useful for creating opportunities in:

- **Remote patient monitoring**. This was largely beneficial for patients in rural and remote regions who previously may not have had ease of access to vital medical services.
- **Store and forward**. A telecommunications technique that allowed for transitioning of systems to transfer information and data from one healthcare provider to another.
- **Consultations**. The new access through audio or video calls improved continuity and quality of care and reduced waiting times for an appropriate treatment, particularly in remote areas.

The blood cancer landscape has changed dramatically since the 19th century. Historical records show that leukaemia (and potentially other types of blood cancers) may have existed for thousands of years, though it remained undiagnosed prior to modern technology.

Although significant advancements have been made in the detection and care of patients with blood cancers, there is opportunity for continued improvement.



Blood film. Image sourced from the Leukaemia Foundation

2.2 PAEDIATRIC BLOOD CANCER

Paediatric or childhood blood cancers differ significantly from adult-onset blood cancers in the biology and aetiology of the condition, prognosis, diagnostic processes, care pathways/treatments, and long-term outcomes. For example, the major subtypes of blood cancers differ dramatically between paediatric and adult settings.

The major blood cancer subtypes for childhood cancers[6] include acute lymphoblastic leukaemia (ALL); acute myeloid leukaemia (AML); non-Hodgkin lymphoma (NHL); Hodgkin lymphoma; juvenile myelomonocytic leukaemia; childhood myelodysplastic syndrome (MDS) and myeloproliferative neoplasms (MPN); and aplastic anaemia. ALL accounts for most paediatric cases (under the age of 15) and is very rarely diagnosed in adults.

Childhood blood cancer remains the most commonly diagnosed childhood cancer at one-third of all cases[7]. Although survival rates have increased dramatically, children still die from childhood AML and survival rates remain poor.

Australia's success in childhood blood cancers is recognised in the use of polymerase chain reaction (PCR[8]) based minimal residual disease testing in childhood ALL. This work, under clinical trial and in collaboration with Australia and New Zealand Children's Haematology and Oncology Group (ANZCHOG), was pioneered by the Children's Cancer Institute and doubled the survival rate of children at highest risk of relapse. This protocol is now ingrained into the standard of care and treatment is highly individualised according to the level of disease or cancer cells remaining in the body.

There are fundamental biological differences in paediatric compared to adult cancers—namely, lower mutational burden and a higher amount of epigenetic dysregulation[9]. The type of cancer, treatment obtained, and individual patient factors increase the risk of later health complications[10].

Although the survivorship of childhood cancers has increased exponentially, survivors are potentially experiencing more late effects[11] than previously seen in their cancer treatment due to living longer. Late effects include recurrence of primary cancer, a secondary or different form of cancer developing, and heart and/or lung damage from cytotoxic treatments.

Given Australia's ageing population and ischaemic heart disease[12] being the leading cause of death, these long-term heart effects may increase disease occurrence and strain on Australia's healthcare system.

CONSULTATION QUESTIONS

Throughout this document you will find consultation questions, relevant to that section. In your response, you are not required to address all questions and should only respond to those relating to your expertise.

1 Should research funding be allocated specific to the:
a. incidence of childhood blood cancers, or
b. long-term health impacts of childhood cancer and cancer treatments?

2 Is there potential to translate paediatric personalised medicine approaches for use in the adult population?

2.3 ADULT BLOOD CANCER INCIDENCE AND RESEARCH ACTIVITY

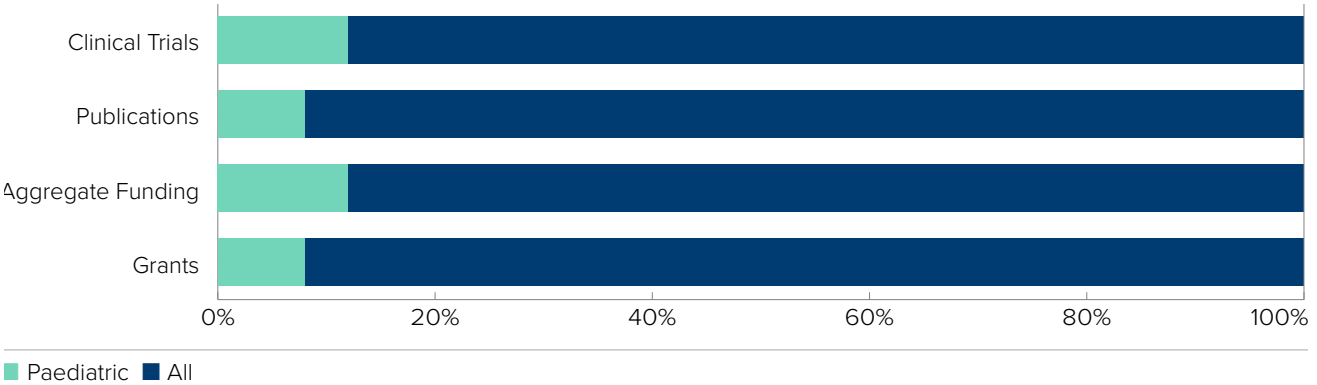
The incidence,[13] diagnosis and treatment of blood cancers and blood disorders differs in adults and across countries as the diseases are genetically very different[14].

Most research and treatment practices in Australia focus on the adult population. This is primarily due to disease occurrence and case numbers.

Data projections[15] completed by the Australian Institute of Health and Welfare show the age diagnosis breakdown for all blood cancers annually. In 2023, 323 childhood blood cancer diagnoses are projected for the 0–14 age group, versus 19,130 diagnoses for ages 15+.

There is also a difference in research funding for the different types of blood cancer. Although 1.7% of all blood cancer cases in Australia are paediatric or childhood cancers, this group accounts for 8.1% of all grants awarded, 11.9% of all aggregate funding, 7.8% of all publications, and 12.4% of all clinical trials completed.

Figure 2.3.1 Proportion of paediatric blood cancer research[16]



3. AUSTRALIA AS A PIONEER OF BLOOD CANCER RESEARCH

Australia has made significant contributions to the blood cancer research and treatment landscape on a global scale. Examples include the molecular response criteria[17] used worldwide to measure the progress of chronic myeloid leukaemia and the use of targeted therapy tyrosine kinase inhibitor.

Another success was the discovery of a method to suppress a specific protein[18] in AML. This protein aided in drug resistance—a common problem among treatments for this subtype of leukaemia.

The Peter MacCallum Cancer Foundation[19] (Melbourne) and Westmead Institute of Medical Research (Sydney[20]) are among the first in the world to manufacture CAR T-cell therapies. One of these drugs, Kymriah[21], uses the body's immune system to fight cancer. The patient's T-cells are extracted from the body, genetically reengineered, and programmed to recognise and destroy cancer cells, before being reimplanted into the body.

State and federal governments have played a significant role in negotiations with pharmaceutical company Novartis to extend the availability of this treatment to eligible patients in specialised tertiary public hospitals via the public health system. This heavily subsidised drug would usually cost more than $500,000 for each patient.

Australia has an opportunity to become a pillar of best practice for blood cancer treatment worldwide if we can accelerate research and capability. Findings could also prove useful for targeting other cancers.

What area of Australian research could have the greatest international impact? **3**

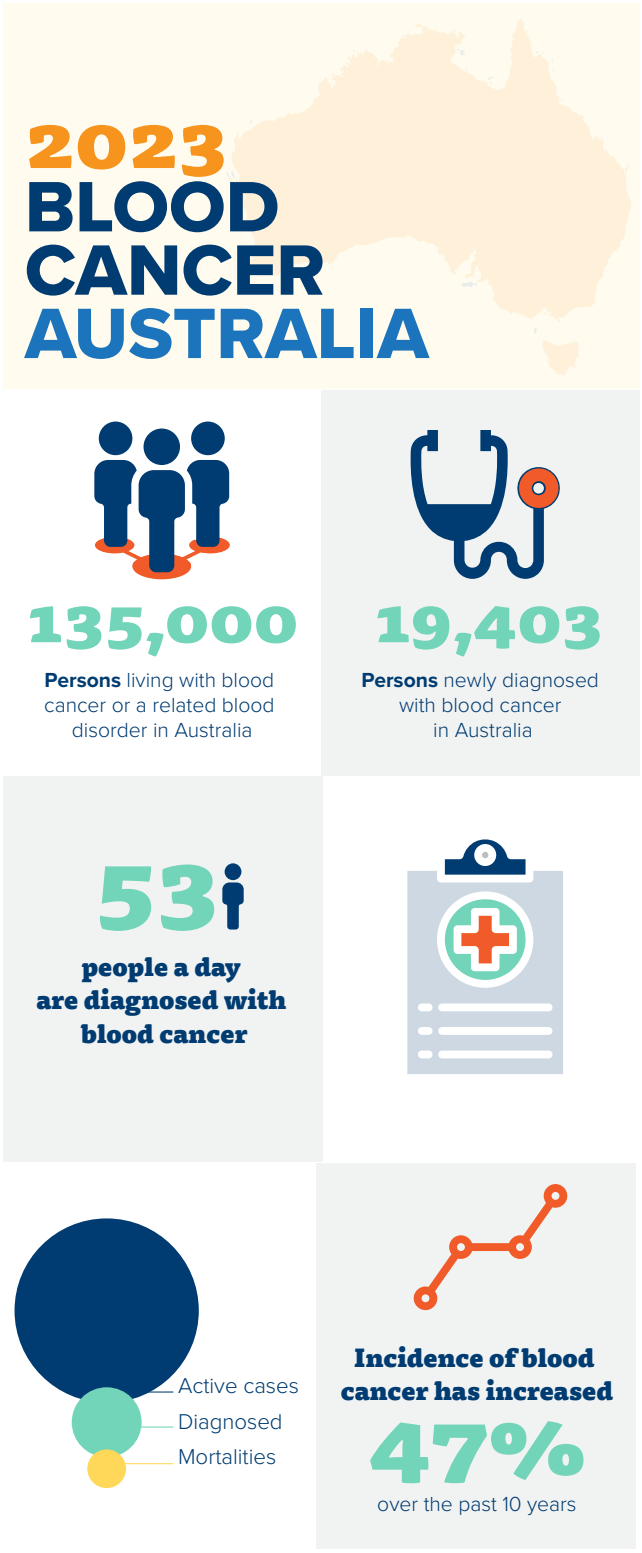


Figure 3.0.1 2023 Blood Cancer Australia[22]

4. BLOOD CANCER RESEARCH: THE GLOBAL CONTEXT

Globally, multiple patient resource organisations and research facilities are active in accelerating blood cancer research and patient outcomes. Multiple roadmaps exist across the United Kingdom[23] (UK) and the United States[24] with an emphasis on National Action Plans to improve the baseline standard of care, access to clinical trials, advance necessary infrastructure, increase political focus, and accelerate research to improve treatment.

The overseas market is highly competitive, particularly in the clinical trial field. Our healthcare system, stable sociopolitical environment, and research reputation make Australia an advantageous place to conduct clinical trial research. Advancing clinical trials in Australasia is one of the main goals of the Australasian Leukaemia & Lymphoma Group (ALLG)—the nation's leading blood cancer clinical trial group.

Major treatment advances overseas include the development of the targeted therapy drug imatinib[25] (Gleevec®) at the Oregon Health & Science University (OHSU) which contributed to target therapy and precision cancer medicine.

The researchers at the OHSU Knight Cancer Institute[26] also assisted in the development of CAR T-cell therapy, which helps the body fight some types of lymphoma and leukaemia.

What are some areas of international research Australia could leverage? **4**

What are examples of international programs that could benefit from collaborations with Australian researchers? **5**

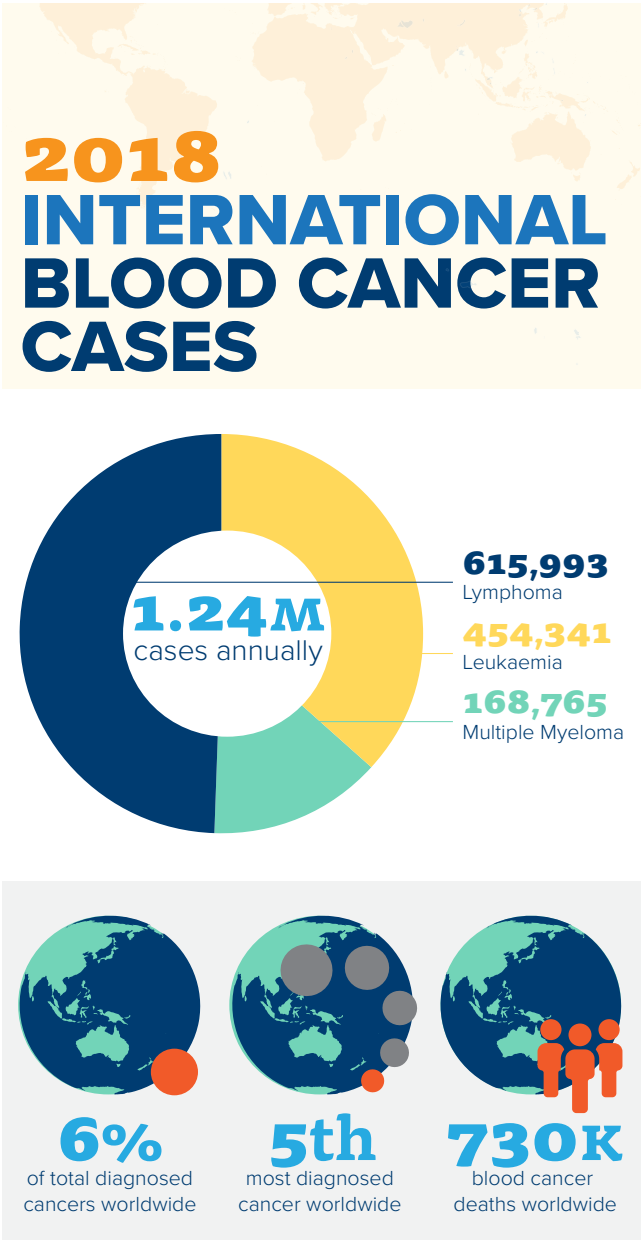


Figure 4.0.1 2018 International blood cancer cases[27]

Is there international research infrastructure Australians could benefit from accessing? **6**

What are the obstacles affecting the advancement of blood cancer research in Australia? **7**

Can this be improved in the short-term future? If so, what methods can be used to fix this? **8**

Image sourced from the Leukaemia Foundation

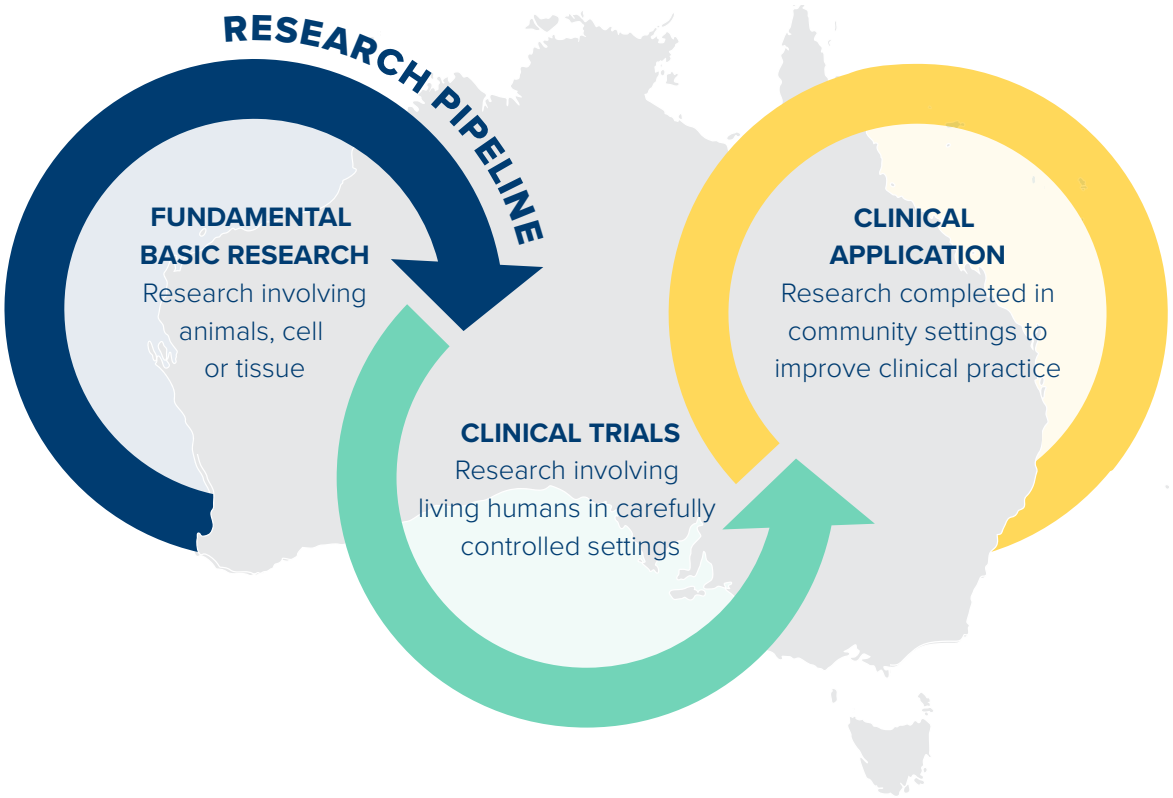
5. CURRENT RESEARCH LANDSCAPE IN AUSTRALIA

5.1 RESEARCH AND TRAINING

In Australia, blood cancer research and training opportunities are funded through a combination of sources, including government funding, philanthropic organisations, non-profit organisations, and community fundraising efforts.

The National Health and Medical Research Council (NHMRC) and Medical Research Future Fund (MRFF) both play significant roles in funding blood cancer research through various schemes including project grants, ideas grants, and programs grants. These grants are open to researchers across different institutions and universities in Australia.

Philanthropic organisations provide grants and funding opportunities specifically focused on blood cancer research. These organisations raise funds through public donations, corporate sponsorships, and fundraising events.



Funding between different types of blood cancers varies. Between 2018 and 2020[28], $14.7 million and $15.2 million were awarded to myeloma and lymphoma research respectively, compared to $67.9 million invested in leukaemia research. Furthermore, specific research areas such as biology and treatment receive most of the funding for blood cancer research, while areas such as aetiology, prevention, early detection, diagnosis and prognosis, and cancer control, survivorship and outcomes research are not invested in as heavily (Figure 5.1.2).

In general, blood cancer research in Australia attracts less funding[29] compared to other cancers when considering the toll on years of life lost. This is despite a significant increase in funding from $18.5 million invested in 2003–2005 and $125 million invested in 2018–2020. Of the 3,502 ‘single tumour’ cancer projects funded in Australia between 2012 and 2020, 5.6% of projects focused on blood cancers.

In the UK, the spread of funding across myeloma, lymphoma and leukaemia follows a similar pattern to Australia—just on a bigger scale. Across the same period of 2018 to 2020[30], funds invested in blood cancer research in the UK were nearly double those invested in Australia. Specifically, ~£12 million (A$22.8 million), and £20 million (A$37.9 million) were invested in myeloma and lymphoma research respectively, and £70 million (A$132.8 million) in leukaemia research—of which, the majority was directed toward biology and treatment.

Figure 5.1.1 Direct funding for ALL cancer research and programs in Australia (2012-2020)[31]

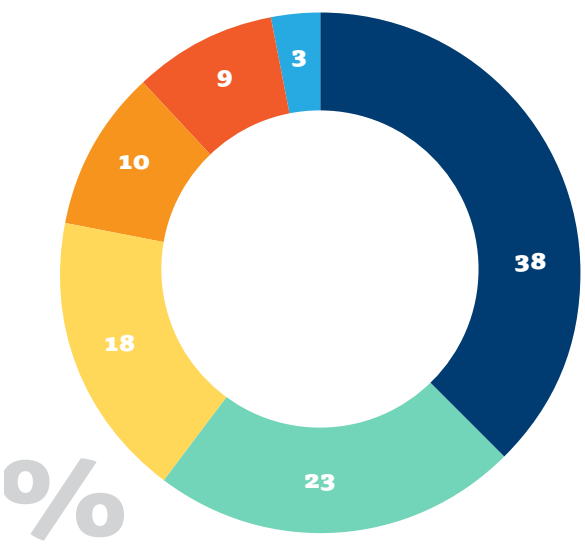
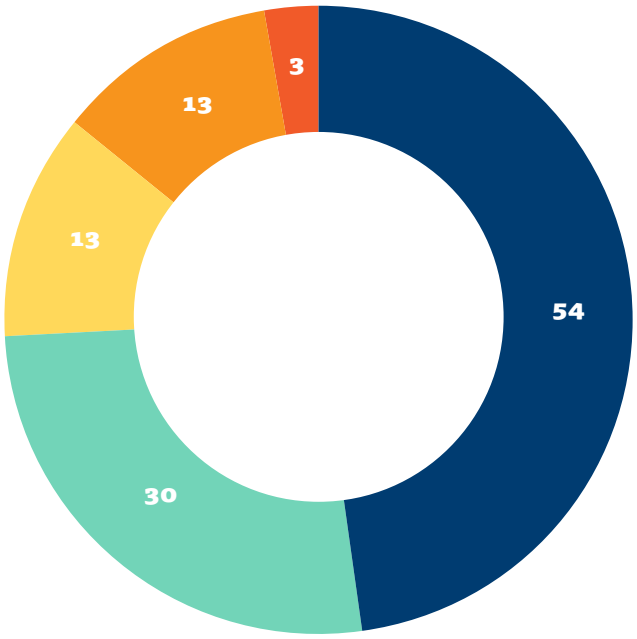


Figure 5.1.2 Direct funding for haematological cancers in Australia (2018-2020)[32]



Treatment research received
Biology research received
Early detection, diagnosis and prognosis received
Aetiology received
Cancer control, survivorship and outcomes research received
Prevention research received

Multiple sources contribute to cancer research funding in Australia including government, philanthropy, cancer foundations, medical institutes, universities, and the NHMRC.

The NHMRC is the largest funder and plays a significant role in funding blood cancer research through various grant schemes. These grants are open to researchers across different institutions and universities in Australia. Between 2018 and 2022 the NHMRC has funded over $86 million for blood cancer research.

The Australian Government's MRFF also contributes substantially to blood cancer research. Since 2013-14[33], the MRFF has allocated more than $314 million in blood cancer research, $30 million in clinical trials, and $80 million for treatment through the Centre of Excellence in Cellular Immunotherapy at Melbourne's Peter MacCallum Cancer Centre.

The Blood Cancer Research Roadmap and First Nations Epidemiology Study to better understand blood cancer within First Nations communities, are fully funded and supported by the Leukaemia Foundation through philanthropic donations. The Government contributed a combined investment of more than $1.75 million to aid the development of the National Action Plan, optimal care pathways, and other activities including clinical guidelines. Additionally, Australian universities and research institutes have dedicated departments and research centres focusing on cancer, including blood cancer, that offer research positions and training programs for scientists and clinicians. As indicated in the 'State of the Nation 2023' and citation analysis, Australia's research community has made significant contribution to this field and are world leaders in blood cancer research.

Figure 5.1.3 Proportion of funding by funding source (ALL Cancer) in Australia (2018-2020)[34]

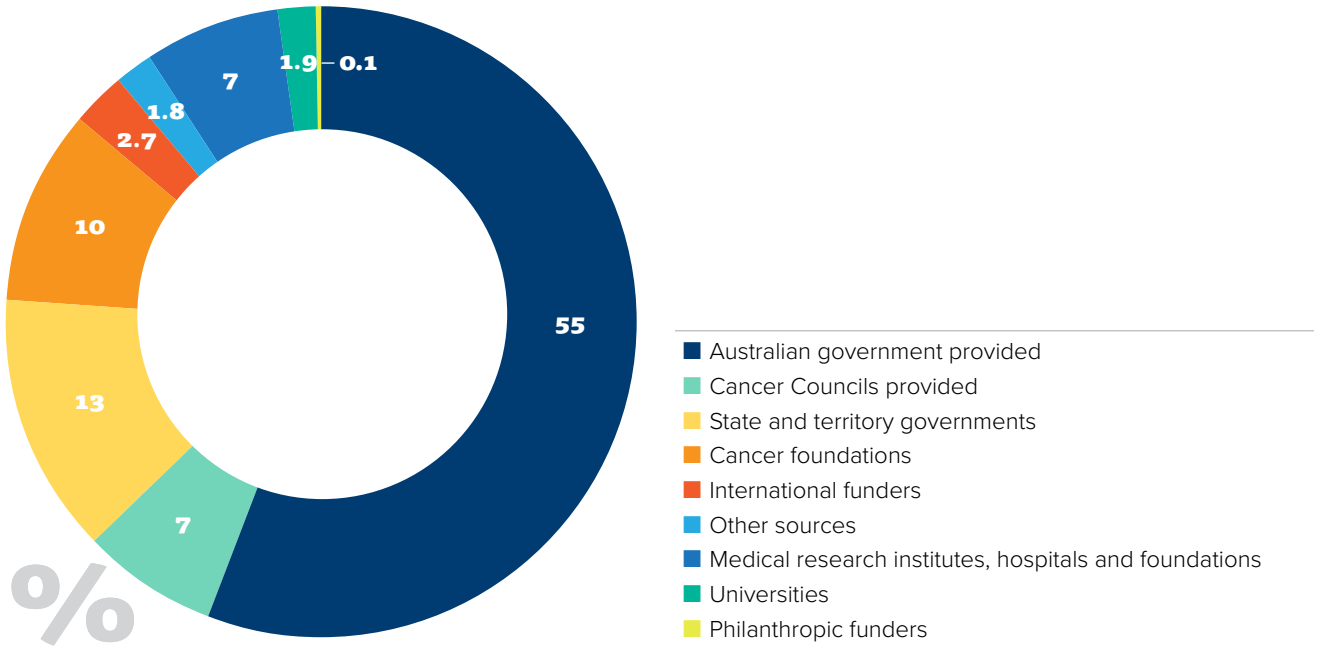


Figure 5.1.4 UK Blood Cancer research spending (figures in million pounds)[35]

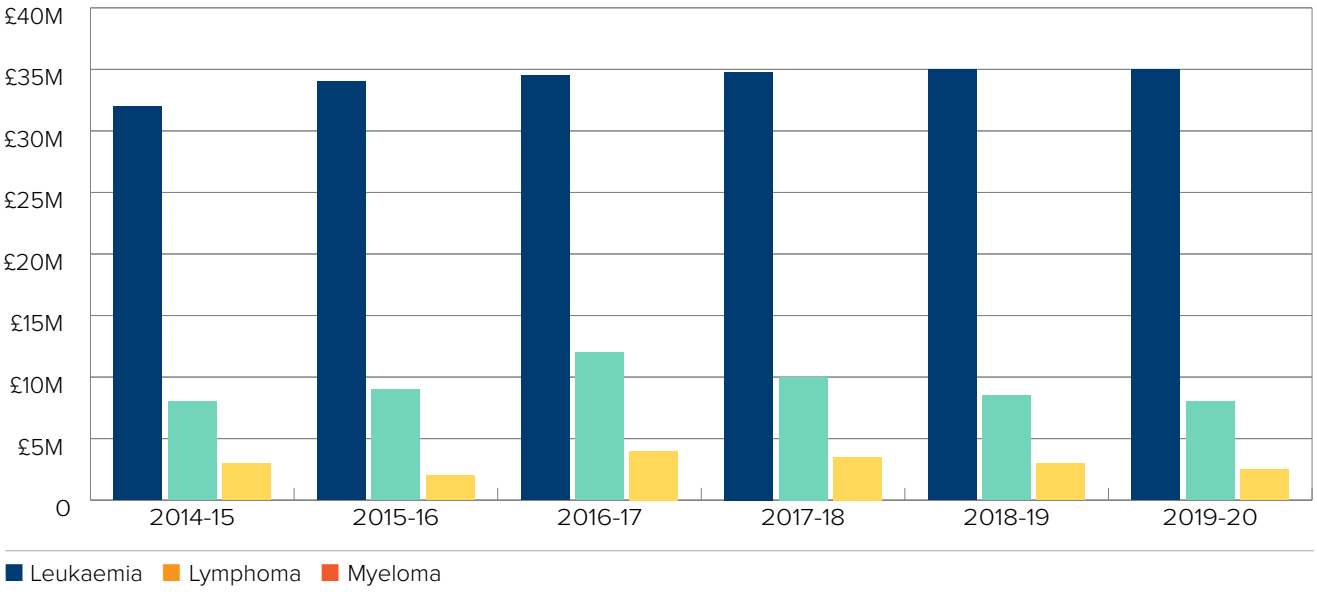


Figure 5.1.5 Proportion of UK blood cancer research funding programs (2019-2020)[36]

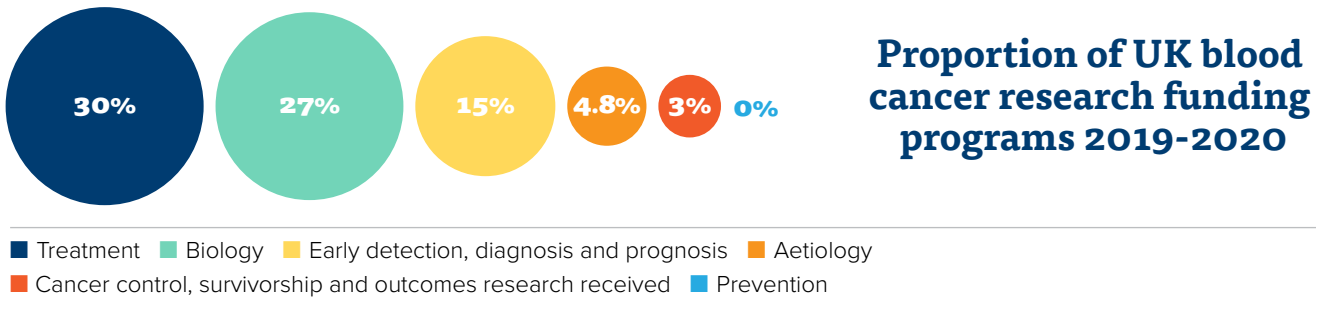
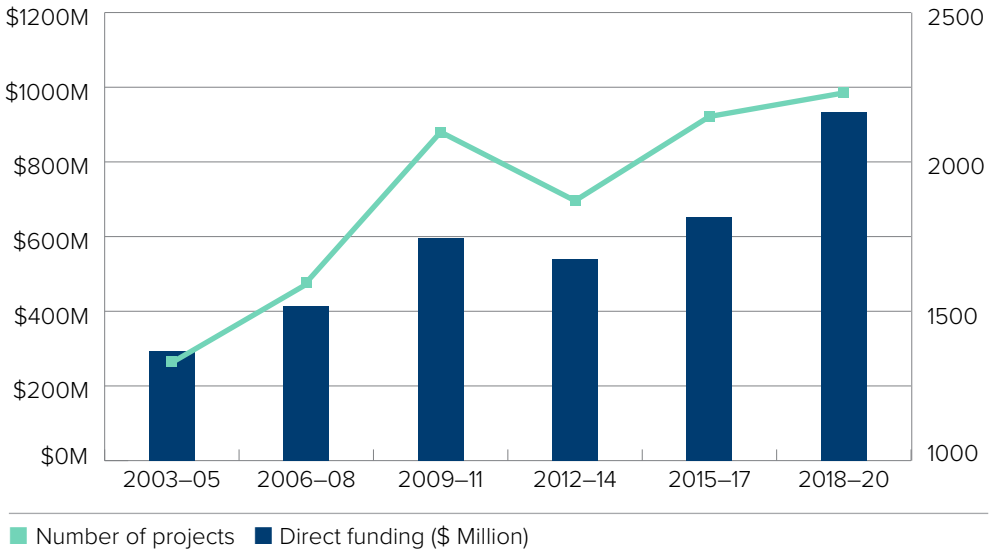


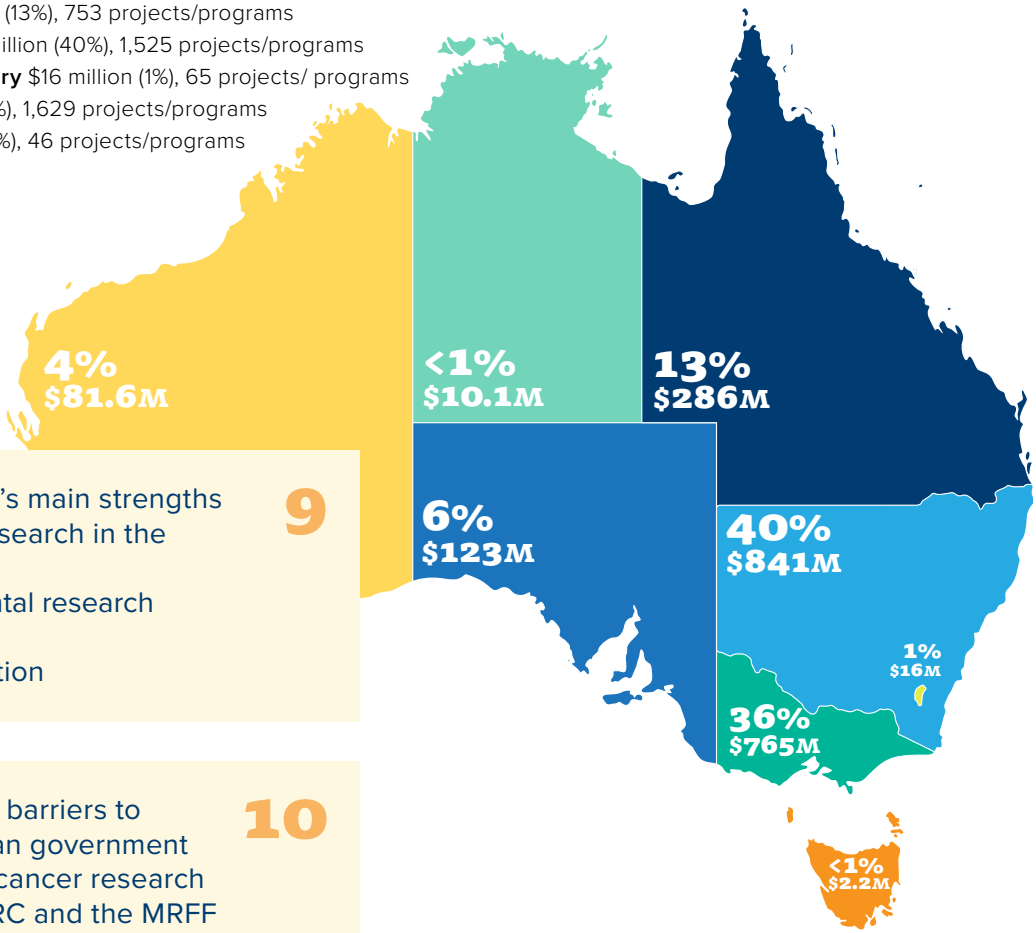
Figure 5.1.6 Direct funding and number of cancer research projects in programs in Australia (ALL cancers)[37]



5.6% of the 3,502 'single tunour' projects funded in Australia 2012-2020 were allocated to blood cancers

Figure 5.1.7 Distribution of funding across Australia (ALL cancers) 2012-2020[38]

- **Western Australia** $81.6 million (4%), 379 projects/programs
- **Northern Territory** $10.1 million (<1%), 17 projects/programs
- **South Australia** $123 million (6%), 399 projects/programs
- **Queensland** $286 million (13%), 753 projects/programs
- **New South Wales** $841 million (40%), 1,525 projects/programs
- **Australian Capital Territory** $16 million (1%), 65 projects/ programs
- **Victoria** $765 million (36%), 1,629 projects/programs
- **Tasmania** $2.2 million (<1%), 46 projects/programs



What are Australia's main strengths in blood cancer research in the following areas?

9

i. Basic fundamental research
ii. Clinical trials
iii. Clinical application

What are the main barriers to obtaining Australian government funding for blood cancer research through the NHMRC and the MRFF for each of the below?

10

i. Basic/fundamental research
ii. Clinical trials
iii. Clinical application

5.2 CLINICAL TRIALS

The ALLG, Australian and New Zealand Myeloma and Related Diseases Registry (ANZMRR) and ANZCHOG all provide platforms for clinicians and researchers to access and participate in clinical trials.

The Australian Trade and Investment Commission, Austrade, has developed a Clinical Trials Industry Capability Report[39] to encourage companies around the world to invest in and establish clinical trials in Australia.

The Australian Commission on Safety and Quality in Health Care have conducted a review of Australia's national Clinical Trials Governance Framework to streamline the clinical trial network and improve the clinical trial environment. This work identified the opportunity for the universal healthcare systems of Australia, the UK, South Korea, and Canada to independently implement a national clinical trials governance framework.

Although there are significant opportunities for big pharmaceutical and medical technology companies to enter the Australian clinical trial landscape, the country's geographical isolation remains a barrier for infrastructure needs and establishment costs.

The strong reputation of Australia's scientific and medical research and use of guidelines (e.g. good clinical practice (GCP) standards, advice from the European Committee for Medicinal Products for Human Use, guidelines from the International Council for Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use) generates confidence in the findings of the clinical trials conducted here.

What sovereign research capability do we need onshore, that can't be imported from overseas? Why? **11**

In what areas of blood cancer research do you consider Australia to be a world leader? **12**

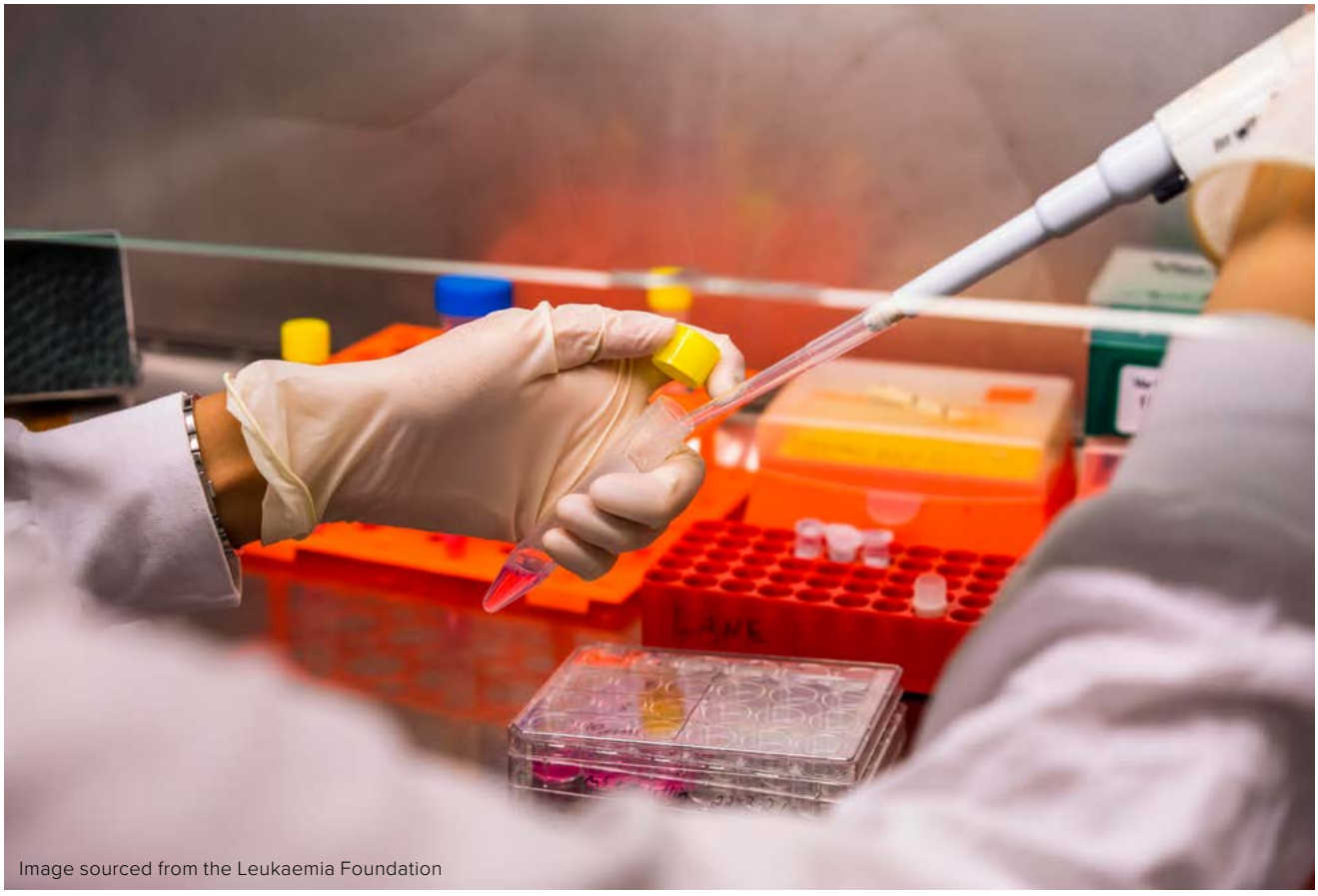


Image sourced from the Leukaemia Foundation

6. POTENTIAL PRIORITY RESEARCH AREAS FOR TREATMENTS AUSTRALIA

An initial set of priorities have been identified through desktop research of Australian and global needs. These determinants are in a draft stage. Further consultation will determine the appropriate opportunities for Australia's research community as guided by the blood cancer community.

Opportunities for further research and clinical advancement in Australia may include:

Epigenetics
Chemical modifications to genetic material that increase or decrease gene expression. Many cancer cells exploit epigenetic modification, whereby oncogenes are up-regulated, and tumour suppressor genes are down-regulated. Drugs/therapies that target epigenetic regulators have shown promise for treatment of blood cancers.

Monoclonal antibodies
A type of immunotherapy which binds to cancer cell and blocks cancer cell signalling (preventing proliferation of cancer cells) or guides targeted treatments to the cancer cell.

Pro-apoptotic agents
Dysregulation of apoptotic pathways can lead to evasion of cell death and proliferation of cancer cells. In some cases, drug/treatment resistance can be attributed to dysregulation of apoptotic pathways. Pro-apoptotic agents directly target apoptotic pathways to induce cell death in cancer cells.

Targeting the microenvironment
Making the cancer cell's environment hostile. The tumour microenvironment can be manipulated in various ways (i.e. anti-angiogenic agents, extracellular matrix targeting to discourage metastasis, target circulating tumour cells, and recruiting/reprogramming the immune system).

Genomics (somatic)
Somatic genomic testing involves the comparison of the genomes of normal and cancer cells to gain an understanding of exactly which errors have caused the cancer. This is used to improve diagnosis to assist in choosing the right treatments (including direction to an appropriate clinical trial) with fewer side effects and better quality of life.

Genomic (germline)
Germline genomic testing involves looking at genomic changes that a person is born with. In rare cases, single gene changes can cause predispositions to certain cancers (including blood cancer) which can be inherited through generations. Genetic changes can also be detected in an individual's chromosome structure (how genes are packaged) which can increase their risk of certain cancers. More recent technologies have also allowed researchers to identify many small variations in an individual's genome (polygenic risk factors), which when taken together, can also greatly influence a person's risk of certain cancers.

Microbiota
Gut microbes stimulate the immune system, breaking down food compounds and synthesising vitamins, enzymes, and amino acids. These microbes can affect the efficacy of chemotherapeutic drugs due to compounding effects on the metabolism and their ability to regulate the tumour microenvironment. These microbes can be used as a predictor for infection, recurrence, Graft-versus-host disease, and death in patients receiving immunotherapy. The specifics of which microorganisms[40] play the key role in these processes is currently up for debate, which is why further research is needed in this area.

Targeted therapy
A type of drug treatment, attacking specific cancer cell features (molecular targets) to stop the cancer growing and metastasising. This is sometimes called molecular therapy or biological therapy.

CAR T-cell therapy
Groundbreaking treatments that take samples of immune cells from the patient and genetically modify them in a laboratory to recognise, seek out, and kill cancer cells, before putting them back into the patient's blood system to fight the cancer. This method is becoming increasingly available.

In addition to those listed above, a broader range of opportunities exist for prioritisation, including:

- The use of existing approved medicines for individualised treatment (precision medicine).
- Advancing our understanding of environmental, lifestyle, and polygenic risk factors for blood cancer, to stratify risk in the population and guide personalised management for prevention and early detection.
- Holistic medicine to consider life before diagnosis as a factor to individualised care.
- Research into promoting a better understanding of the impact of blood cancer on Aboriginal and Torres Strait Islander communities, with translatable actions for better, culturally sensitive care.

In addition to the challenge of remote and rural communities (as indicated in the MMM[41] model), how can this vulnerability be adequately addressed? **13**

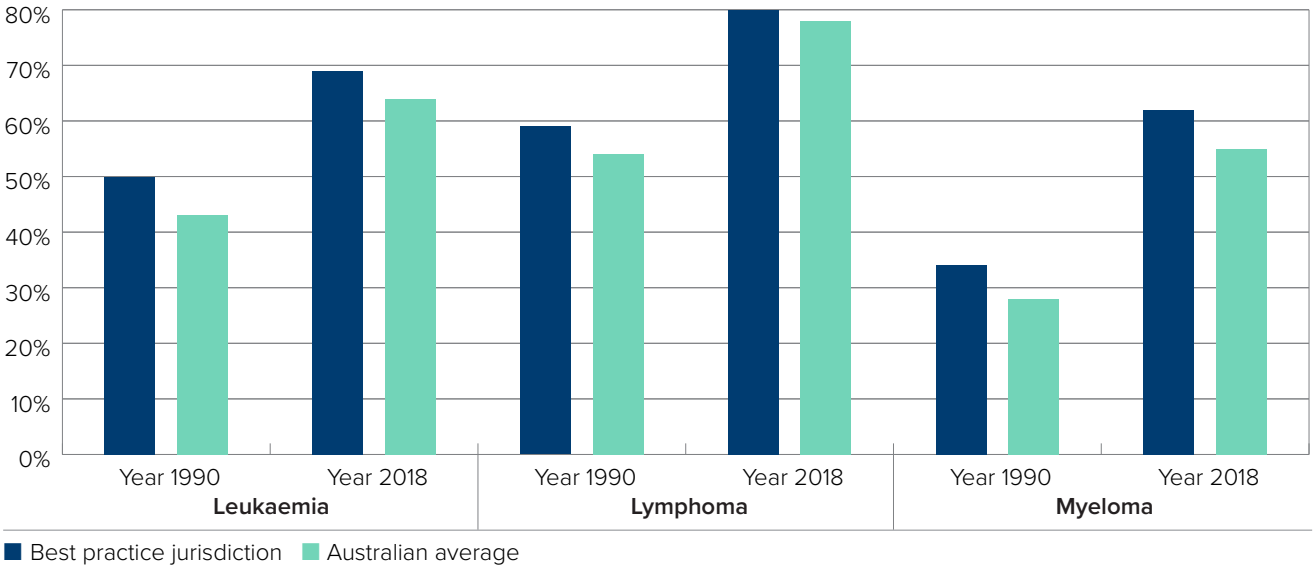
Are the listed priorities in section 6 correct? If not, what should the priority research areas be? **14**

Of these areas, which is most important and why? **15**

6.1 OPPORTUNITIES

The acceleration and prioritisation of fundamental and applied research will have a cumulative effect on case outcomes. The opportunities that arise from the creation of a research roadmap are linked to raising the optimal care standard. Achieving better outcomes for blood cancer has wider benefits including translation across multiple healthcare disciplines.

Figure 6.1.1 Five-year survival outcomes by major blood cancer sub-type (1990–2018) comparing the national average to the best practice jurisdiction.[42]



There are distinct barriers to an optimal research landscape, such as limited funding, poor or inadequate infrastructure, and inadequate organisational support[43]. This affects researchers, clinicians, and patients in various modes and quantifiers. Planning, implementation, and decision-making processes by governments of all levels are crucial in the success of a national scheme.

6.2 GEOGRAPHICAL SCALE

The geographical scale of Australia leads to disparities in healthcare, with services less accessible for rural, remote, and very remote communities. To quantify this problem, the Modified Monash Model (MMM[44]) was developed to define whether a place is metropolitan, rural, remote or very remote. It assigns a category based on the Australian Statistical Geography Standard-Remoteness Areas framework and is updated in accordance with the national census every five years.

This hurdle calls for further research into disparity of care, telehealth, and delivery of care across the country from both an adult and a paediatric perspective. This geographical barrier presents both a challenge and an opportunity for Australia to become a world leader in remote healthcare delivery, with consultancy opportunities across the globe.

What is the strategic planning process behind your organisations grant applications? **16**

Is there a national funding strategy regarding cancer research, or blood cancer research more specifically? **17**

Is a specific metric used to determine how much funding is allocated to a specific healthcare concern? **18**

Is the infrastructure required for blood cancer research spread equally across the nation? If not, could this spread be improved, and on what basis? **19**

Is the current alignment of funding an obstacle to accelerating blood cancer research in Australia? **20**

What research infrastructure is currently in place, and is relevant to blood cancer researchers? What more is required? **21**

Is there opportunity for collaborative biorepositories? What benefits would this bring to Australian blood cancer research? **22**

How would you describe Australia's capacity for blood cancer research? **23**

7. EMERGING TECHNOLOGIES

Digitalisation and data intensive activities are driving rapid advances in technology. The healthcare industry has the opportunity to utilise these developments across small- or large-scale programs. Overseas, the use of artificial intelligence technology and deep learning has been embraced into clinical practice including note taking, algorithms recognising patterns of malignant tumours, administrative processes in clinical trials, and precision medicine.

Machine learning[45] has been used in healthcare for many years to predict disease outcomes. This is particularly useful in cancer staging, diagnosis, and determining treatment plans based on characteristics found in the cancer cells and images (computer-aided detection).

How would you describe the ability of Australian researchers to collaborate with each other? What are the enablers and barriers to these collaborations? **24**

How would you describe the ability of Australian researchers to collaborate with researchers internationally? What are the enablers and barriers to these collaborations? **25**

What are other examples of emerging technologies that will impact the future of blood cancer research? **26**

How will these technologies impact: **27**
a. research
b. clinical practice
c. public health outcomes?

Image sourced from the Leukaemia Foundation

8. CONCLUSION AND IMPLEMENTATION

The Research Roadmap will consider how to advance research and patient care in priority areas and look to leverage existing capability and strengths in Australia to achieve impact both locally and on a global scale.

A framework for implementation will be proposed within the roadmap. This framework will assess and monitor the implementation and effectiveness of evidence-based interventions.

In conjunction with the data collected from responses to this consultation paper, the Academy will conduct a series of surveys and webinars to explore the current research landscape in Australia.

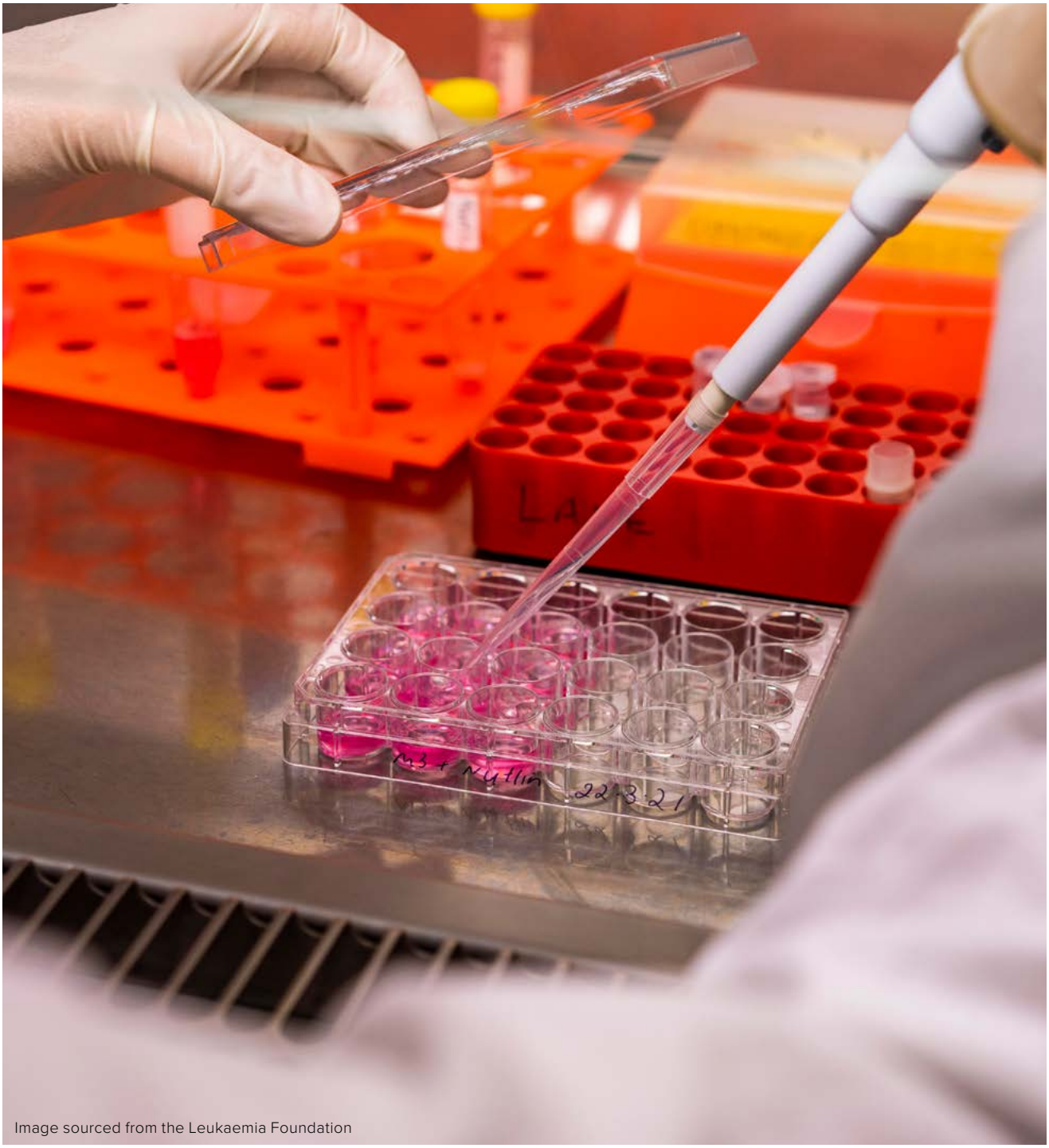


Image sourced from the Leukaemia Foundation

8.1 CONSULTATION QUESTIONS

1. Should research funding be allocated specific to the:
   a. incidence of childhood blood cancers, or
   b. long-term health impacts of childhood cancer and cancer treatments?
2. Is there potential to translate paediatric personalised medicine approaches for use in the adult population?
3. What area of Australian research could have the greatest international impact?
4. What are some areas of international research Australia could leverage?
5. What are examples of international programs that could benefit from collaborations with Australian researchers?
6. Is there international research infrastructure Australians could benefit from accessing?
7. What are the obstacles affecting the advancement of blood cancer research in Australia?
8. Can this be improved in the short-term future? If so, what methods can be used to fix this?
9. What are Australia's main strengths in blood cancer research in the following areas?
   i. Basic/fundamental research
   ii. Clinical trials
   iii. Clinical application
10. What are the main barriers to obtaining Australian government funding for blood cancer research through the NHMRC and the MRFF for each of the below?
    i. Basic/fundamental research
    ii. Clinical trials
    iii. Clinical application
11. What sovereign research capability do we need onshore, that can't be imported from overseas? Why?
12. In what areas of blood cancer research do you consider Australia to be a world leader?
13. In addition to the challenge of remote and rural communities (as indicated in the MMM model), how can this vulnerability be adequately addressed?
14. Are the listed priorities above correct? If not, what should the priority research areas be?
15. Of these areas, which is most important and why?
16. What is the strategic planning process behind your organisations grant applications?
17. Is there a national funding strategy regarding cancer research, or blood cancer research more specifically?
18. Is a specific metric used to determine how much funding is allocated to a specific healthcare concern?
19. Is the infrastructure required for blood cancer research spread equally across the nation? If not, could this spread be improved, and on what basis?
20. Is the current alignment of funding an obstacle to accelerating blood cancer research in Australia?
21. What research infrastructure is currently in place, and is relevant to blood cancer researchers? What more is required?
22. Is there opportunity for collaborative biorepositories? What benefits would this bring to Australian blood cancer research?
23. How would you describe Australia's capacity for blood cancer research?
24. How would you describe the ability of Australian researchers to collaborate with each other? What are the enablers and barriers to these collaborations?
25. How would you describe the ability of Australian researchers to collaborate with researchers internationally? What are the enablers and barriers to these collaborations?
26. What are other examples of emerging technologies that will impact the future of blood cancer research?
27. How will these technologies impact:
    a. research
    b. clinical practice
    c. public health outcomes?

9. APPENDIX: FURTHER REPORTS

ZIMMERMAN REPORT

In 2020, the Standing Committee on Health, Aged Care and Sport conducted an inquiry into the approval processes for new drugs and novel medical technologies in Australia. The report 'The New Frontier – Delivering better health for all Australians'[46] is the result of that inquiry.

The Committee heard from clinical experts, patient groups and their families who urged support for a more flexible system to provide timely access to the latest medicines, devices, and treatments.

A big challenge facing the existing system is the trend towards precision medicine. This emerging approach to disease treatment and prevention considers individual variations in genes, environment, and lifestyle for each patient. This development was not envisaged when the current regulatory and reimbursement system was designed and legislated.

The Committee expressed concern about the complexity of the approvals system for medicines and medical technologies, including the interaction between the Commonwealth, states, and territories.

Recommendations made by the Committee in the report include:

1. Creation of a Centre for Precision Medicine and Rare Diseases within the Department of Health, to provide advice on research priorities, education and training for clinicians and patients, and the development of a comprehensive horizon scanning unit for new medicines and novel medical technologies.
2. Establish a new pathway for cell and gene therapy to simplify Health Technology Assessment (HTA) processes.
3. Reforms that will strengthen the central role of patients in the assessment system. The Committee heard from patients and their families about the need for more patient involvement in the approvals decision-making process for new drugs and novel medical technologies. The patients have a crucial perspective on what treatments work best for them, including important lifestyle benefits, but this has traditionally not been given enough attention within the regulatory and reimbursement system.
4. Changes to encourage companies to enter the Australian market with their products and technologies. This includes changes to the fee structure for applications to the Therapeutic Goods Administration and HTA processes—particularly for orphan drugs and smaller companies, including Australian start-ups. Some medicines and technologies (particularly orphan drugs for rare diseases) are available overseas and not in Australia for commercial reasons.
5. The creation of an annually capped fund with clear and transparent eligibility rules to provide funding for application by patients, clinicians, and non-profits, where there is no realistic prospect of a company serving as a sponsor.
6. Changes to streamline the system to ensure Australia is a more attractive location for clinical trials. This includes the immediate harmonisation of ethics and governance approvals into one online platform and the establishment of a national clinical trials register. Comparatively, Australia is a top-tier country for trials, not only for developing our own research capacity but, more importantly to ensure early access to life changing drugs and technologies.
7. Support for stronger and more collaborative R&D. Patient groups advocated strongly for the repurposing of existing medicines to treat alternate disease or conditions. Required a more flexible vision for the future and recommends the establishment of a new pathway that incentivises the repurposing of drugs for all diseases.

NATIONAL STRATEGIC ACTION PLAN FOR CANCER[47]

Recommendations were grouped into four areas:

1. Empower patients and their families
2. Achieve best practice
3. Accelerate research
4. Enable access to novel and specialised therapies.

Priorities:

- **Action 3.1** Identify priority areas for research funding that deliver greater impact through a Blood Cancer Research program.
- **Action 3.2** Improve value and use of real-world data for blood cancer patients.

Actions to accelerate research breakthroughs by increasing research and funding through a Blood Cancer Research Program are some of the most critical aspects of the National Action Plan.

STATE OF THE NATION: BLOOD CANCERS IN AUSTRALIA[48]

The first report in 2019 brought together Australian governments and the blood cancer community to table a long-term plan to improve outcomes for people impacted by blood cancer.

Following the first report (released in 2019):

- National Blood Cancer Taskforce established
- National Strategic Action Plan for Blood Cancer
- Development of 11 Optimal Care Plans (OCPs) (six have been delivered with the remaining five in development).

The second report released in February 2023 notes the proposal of a national cancer data ecosystem. This allows opportunity for addressing systematic data issues that are instilling barriers in the current blood cancer research landscape. Simultaneously, the improvements this will bring can be translated to health service delivery as a whole and therefore patient outcome.

Australia is in the process of developing a future-focused ten-year Australian Cancer Plan with associated ten-, five- and two-year actions and goals. This will act as a national framework to accelerate best practice. This plan, in conjunction with the OCPs targets priority population groups of:

- People living in rural and remote areas
- Aboriginal and Torres Strait Islander people
- LGBTIQA+ people
- People from culturally and linguistically diverse backgrounds
- People living with a disability
- People living in low socioeconomic areas
- People living with a mental illness
- Older Australians
- Adolescents and young adults
- Children.

The Australian Cancer Plan has six strategic objectives:

1. Maximising cancer prevention and early detection
2. Enhanced consumer experience
3. World-class health systems for optimal care
4. Strong and dynamic foundations
5. Workforce to transform the delivery of cancer care
6. Achieving equity in cancer outcomes for Aboriginal and Torres Strait Islander people.

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