State of the Nation: Blood Cancers in Australia Report 2023

Final Report to Leukaemia Foundation

February 2023

Insight Economics
Public Policy Corporate Strategy

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Executive Summary

In 2019, the Leukaemia Foundation launched the first ever *State of the Nation: Blood Cancer in Australia* report. This report set a goal that by 2035 there would be zero lives lost to blood cancer. In practice, this would mean that by 2035 there are zero preventable deaths, made possible by timely and equitable access to best practice treatment. It would also mean, by 2035, that all blood cancer survivors and their families have timely access to the information and supportive care services they need to enjoy a high quality of life.

The first State of the Nation report (SoTN) brought Australian governments and the blood cancer community together around a long-term plan to improve outcomes for people impacted by blood cancer. For example, following the launch on the first SoTN in 2019, the Australian Government established a National Blood Cancer Taskforce to lead the development of a National Strategic Action Plan for Blood Cancer (NAP). The National Blood Cancer Taskforce was funded to develop eleven Optimal Care Plans (OCPs) for blood cancer; six have been delivered to date with a further five in development. Funding was also provided to the National Blood Cancer Taskforce for the development of a national approach to the creation of Australian clinical guidelines for blood cancer; this work is underway today. Added to this, government acknowledged the need for action to improve access to therapies. Since the launch of the first SoTN, 16 additional medicines have been recommended for listing on the PBS and $80 million in funding was provided to establish Chimeric Antigen Receptor T-Cell (CAR T-cell) therapies in Australia. In addition, the House of Representatives Standing Committee on Health, Aged Care and Sport (the Zimmerman report) supported the first SoTN recommendations for the establishment of a program to improve evidence development and access to therapies (a Right to Trial Program), as well as wider health technology assessment (HTA) and regulatory reforms, which are now underway.

Alongside blood cancer specific actions, the first SoTN has helped to catalyse a series of systemic, multilateral reforms to the health system to improve outcomes for people living with blood cancer. Many ideas of the first SoTN, including recommendations for improved consumer navigation, a national cancer data ecosystem, performance reporting, and GP education have been taken up by the Australian Cancer Plan (ACP).

While important progress has been achieved, even against the backdrop of the COVID-19 pandemic, there remains important work to be done to realise the zero by 2035 vision.

More work is needed to deliver the initiatives identified in the first SoTN and the NAP, many of which have been accepted and endorsed by government. Work is also needed to ensure the significant national reform efforts — such as the ACP and the HTA review — are developed in such a way that they deliver the step-change improvements needed to improve outcomes for blood cancer patients across Australia.

Thus, it is timely to take stock of progress to date and refocus the agenda on the key priorities for action over the next horizon.

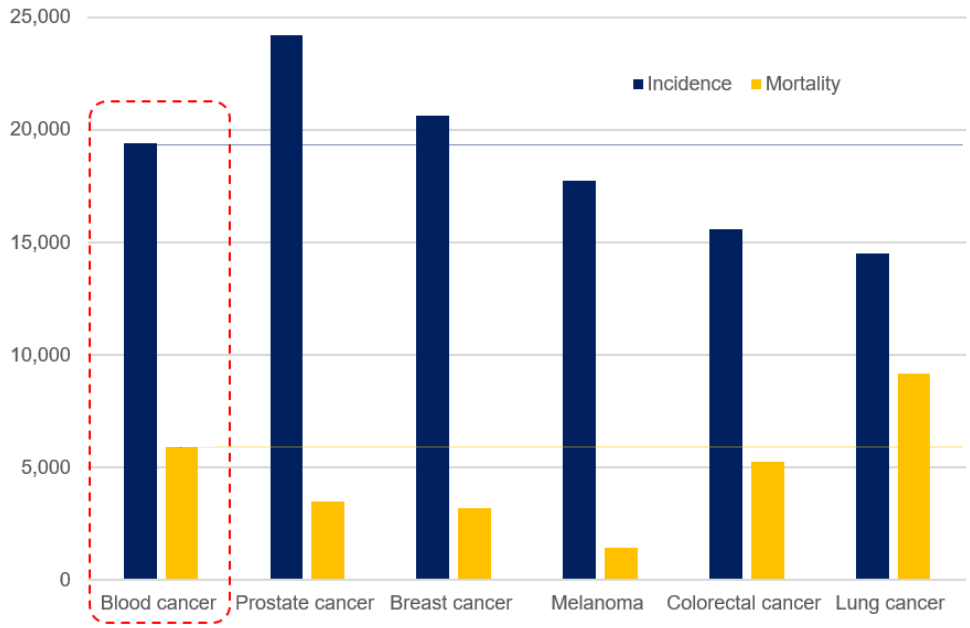
This report updates key evidence from the first SoTN. Specifically, it provides an update of the People Living with Blood Cancer survey, an update of incidence and mortality projections based on latest available cancer registry data, and an update of key stakeholder perspectives regarding the main priorities for action. Remarkably, more than 4,600 people living with blood cancer responded to the consumer survey – a 40 per cent uplift on the previous survey which serves to highlight the continued importance of this policy agenda to Australian communities.

Synthesising these updated data and perspectives, this report summarises the major achievements to date against each strategic objective set out in the first SoTN and identifies key priorities for the next implementation horizon of the zero by 2035 strategy.

Blood cancer remains a significant priority for Australian communities, with unique challenges for patients and their families

Cancer registry data show that blood cancers remain among the most prevalent and significant diseases impacting Australian communities today; more than 19,400 people are diagnosed each year and more than 135,000 blood cancer survivors are living across Australia. Blood cancer is the third most commonly diagnosed type of cancer and the second leading cause of cancer death after lung cancer (Figure 1).

Figure 1: Incidence and mortality of blood cancer in Australia – benchmarking AIHW 2022 estimates



Source: AIHW, 2022, Cancer Data in Australia, incidence projections and mortality count projections (National Mortality Database).

Critically, people living with blood cancer and their loved ones face intense, multi-month treatment regimens that often require patients to relocate from their homes to specialist centres. This can place families at higher risk of financial strain, depression and anxiety than other cancers. Blood cancer survivors are also acutely vulnerable in a post-COVID-19 environment with long-term immunosuppression risks.

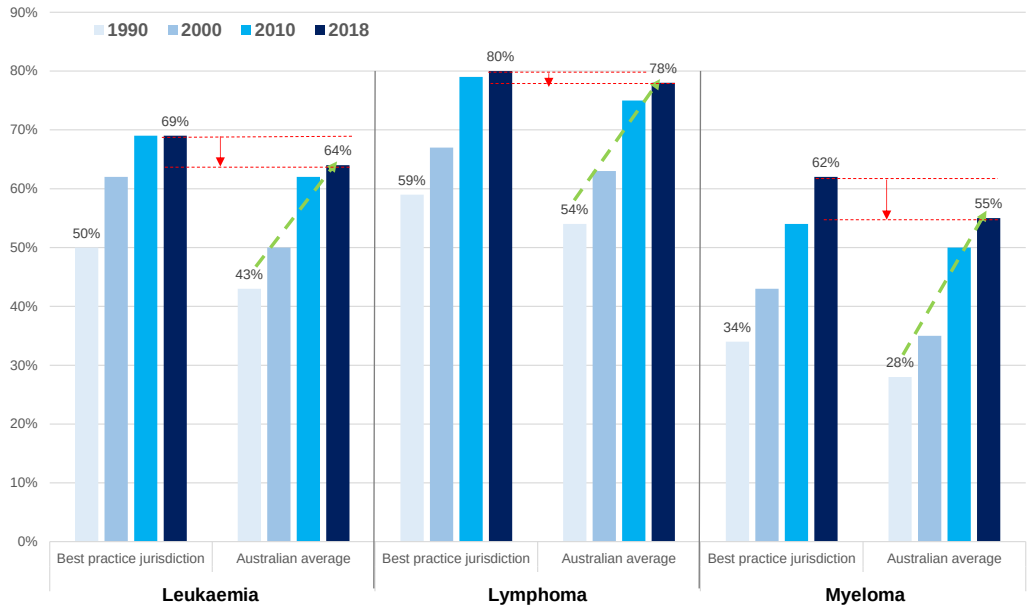
Unlike other cancers, blood cancers cannot be prevented or screened. This means that government strategies must be focused on increased funding for blood cancer research and improving equitable access to novel therapies:

- *Increased funding for blood cancer research* — Funding for blood cancer research is needed to better understand disease biology, which can enable prevention and screening of blood cancers as well as breakthroughs in treatment. In spite of dramatic improvements in survival across many blood cancer sub-types, including in particular paediatric blood cancers, many blood cancers remain low survival cancers. Survivors can also face significant off-target effects from their treatment, severely compromising their quality of life well beyond the completion of active treatment. More work is needed to understand the underlying causes of blood cancers to improve prevention, early detection and breakthroughs in curative therapies.

- *Policy reforms to improve adherence and access to clinical best practice treatment and supportive care* — The first SoTN estimated that between 13 per cent and 30 per cent of blood cancer deaths may be preventable. Available cancer registry data at that time, which was based on reporting over the 2011-2015 period, showed mortality would be reduced by 13 per cent if best practice outcomes by sex, age and blood cancer subtype were realised in every jurisdiction in Australia. Expert opinion at that time (2018) estimated it could be higher — in the order of 30 per cent. The latest available cancer registry data today, which reports outcomes over the 2015-2019 period, confirms the expert opinion was a more accurate assessment of the gap. The latest available cancer registry data show if best practice outcomes were achieved, potentially up to 29 per cent of blood cancer mortality could be prevented. This underscores the conservative estimates of the first SoTN and underlines the need for reforms to improve consistent access to best practice treatment.

Thus, while there have been significant improvements in survival nationally over the past two decades, there remains significant variation in outcomes depending on where a patient is treated. To illustrate this point, Figure 2 shows a historical perspective of 5-year relative survival outcomes for patients nationally compared to a jurisdiction with the best weighted average survival outcomes across all age cohorts, sex and blood cancer sub-type.

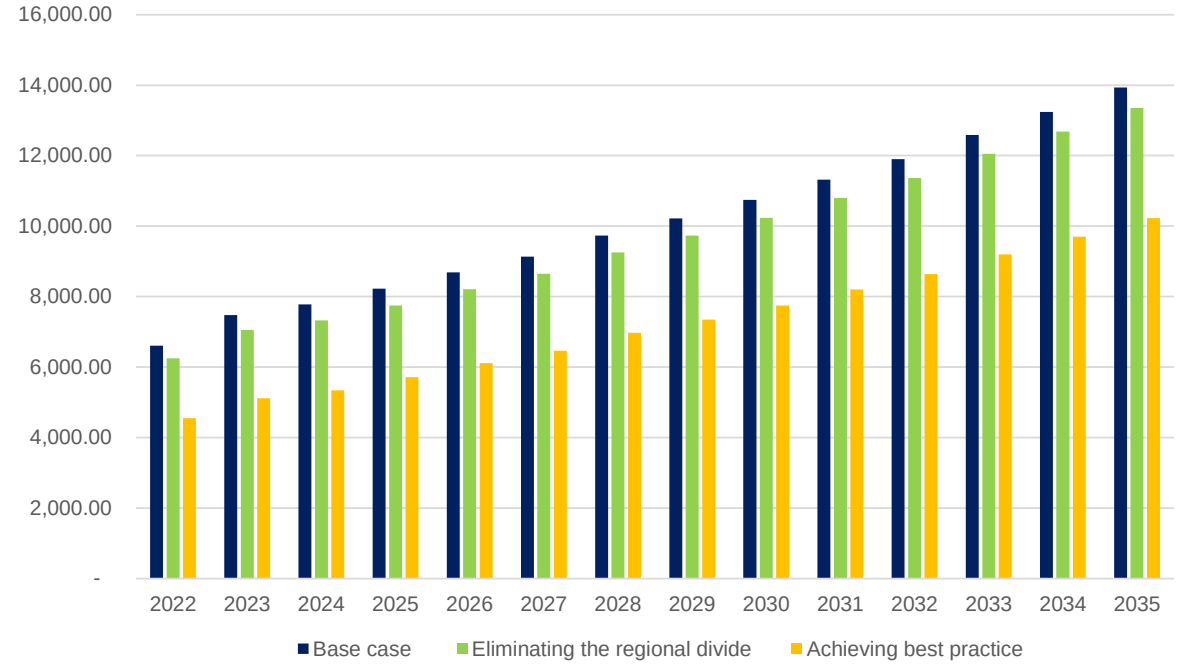
Figure 2: 5-year relative survival outcomes by major blood cancer sub-type (1990-2018) – comparing the national average to the best practice jurisdiction



Source: Analysis of AIHW Cancer database and Cancer Council data.

When variation in outcomes are eliminated for each cohort (stratified by age, gender, blood cancer sub-type and region) based on latest available cancer registry data, this shows that up to 29 per cent of projected blood cancer mortality could be prevented. Over the 2023-2035 (inclusive) horizon, this translates into more than 38,200 preventable deaths or roughly 2,900 deaths per annum on average (Figure 3).

Figure 3: Reducing preventable mortality – benefit potential from achieving best practice



Source: Analysis of state cancer registry and AIHW data.

Key progress in the implementation of actions for the zero by 2035 strategy

The zero by 2035 strategy proposed a long-term ambition to reduce mortality from blood cancer and improve the quality of life of people impacted by blood cancers. A detailed plan for action organised against four strategic objectives was identified. The four key strategic objectives were:

- Empowering people living with blood cancer and their loved ones
- Catalysing health services reform
- Accelerating research
- Enabling access to novel and specialised therapies.

Achieving these strategic objectives requires significant, systems-level reform, which is the product of sustained execution of a range of inter-related actions and advocacy for change.

The review finds that important progress has been made against each of the key strategic objectives since the launch of the first SoTN in 2019 (Figure 4), even against the backdrop of the COVID-19 pandemic.

- Australia's first ever National Blood Cancer Taskforce was established, which led the development of a NAP. The NAP set out a long-term agenda for reform, which was endorsed by government and received initial funding for a sub-set of Phase 1 actions
- The Taskforce led the development of six additional OCPs (with a further five now funded by government) and received federal funding for the development of a national approach to the creation of Australian clinical guidelines for blood cancer
- Important ideas identified in the first SoTN and NAP have been incorporated into the draft ACP which recognised that actions would benefit all cancer patients. The most significant reforms adopted include:

  - A national, integrated Consumer Navigation Service that would improve the availability of disease information to patients and carers and improve referral of patients to consumer navigation support from diagnosis
  - GP education on the signs and symptoms of cancer, to be organised around optimal care pathways
  - A national cancer data ecosystem, which would provide the basis for performance reporting metrics of quality and safety
  - The development of a national approach to Patient Reported Outcome measures for cancer
  - Development of a National Comprehensive Cancer Center Network and models of care to improve the use of multidisciplinary teams (MDTs) and regional service delivery
- Access to new medicines has improved, with 16 new medicines listed on the PBS and $80 million in funding for CAR T-cell therapy alongside approvals for three therapies, which are now available at eight sites nationally
- The House of Representatives Standing Committee on Health, Aged Care and Sport led an Inquiry into approval processes for new drugs and novel medical technologies in Australia (Zimmerman report). The Inquiry supported first SoTN recommendations for the establishment of programs to improve evidence development and access to therapies (Right to Trial Fund), as well as wider health technology assessment and regulatory reform, which are now currently underway
- The Leukaemia Foundation has funded the development of a Research Roadmap, which will provide a framework for investment by government and the community into areas of high-impact blood cancer research
- The Leukaemia Foundation has funded an epidemiological study of blood cancer in Aboriginal and Torres Strait Islander communities, to address data gaps and support the development of a strategy to improve outcomes for these communities.

At the same time, there is much work to be done to see these first steps of progress translate into real change for patients and their loved ones.

Figure 4: On the path to zero by 2035 – progress and next steps by strategic objective

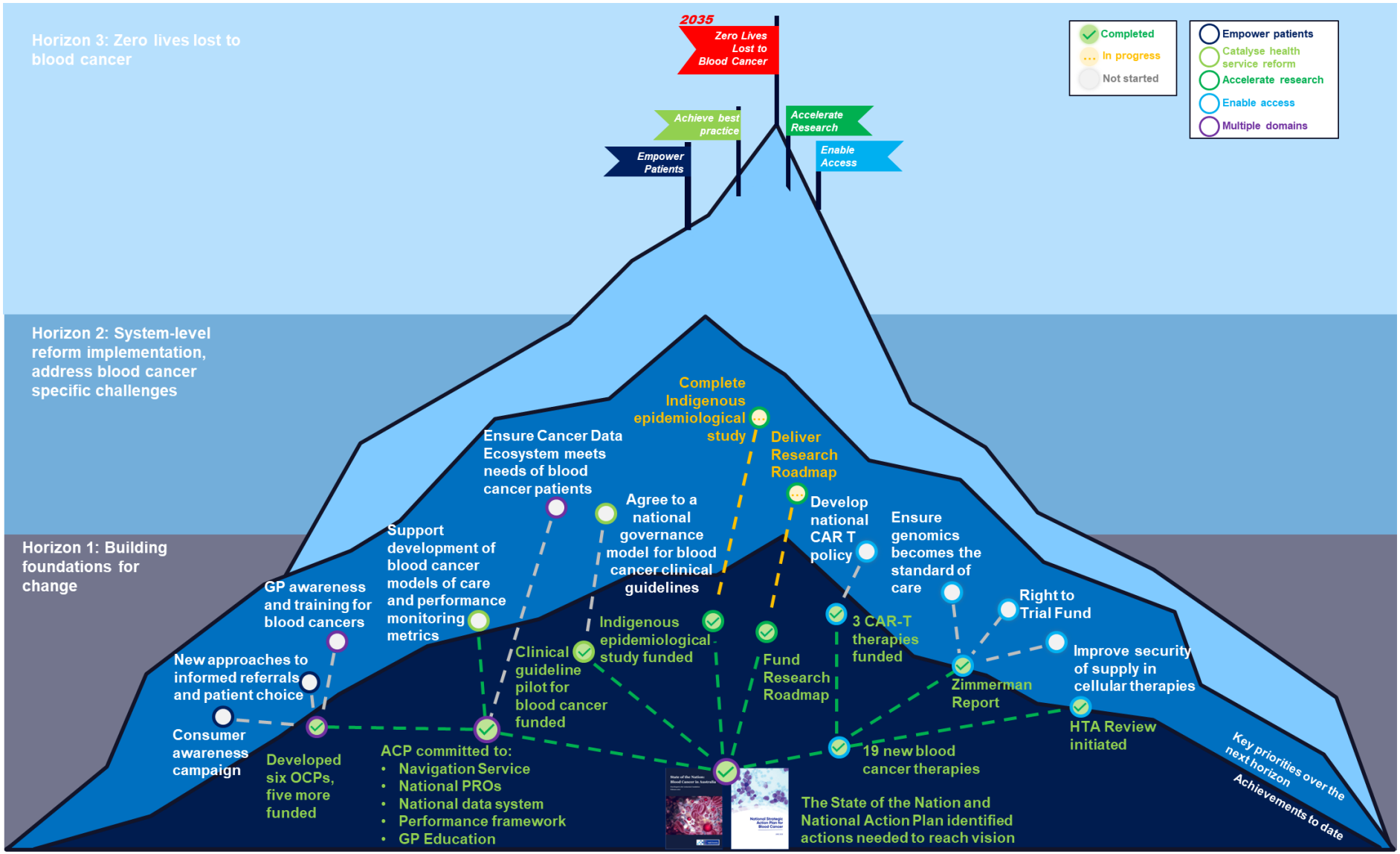
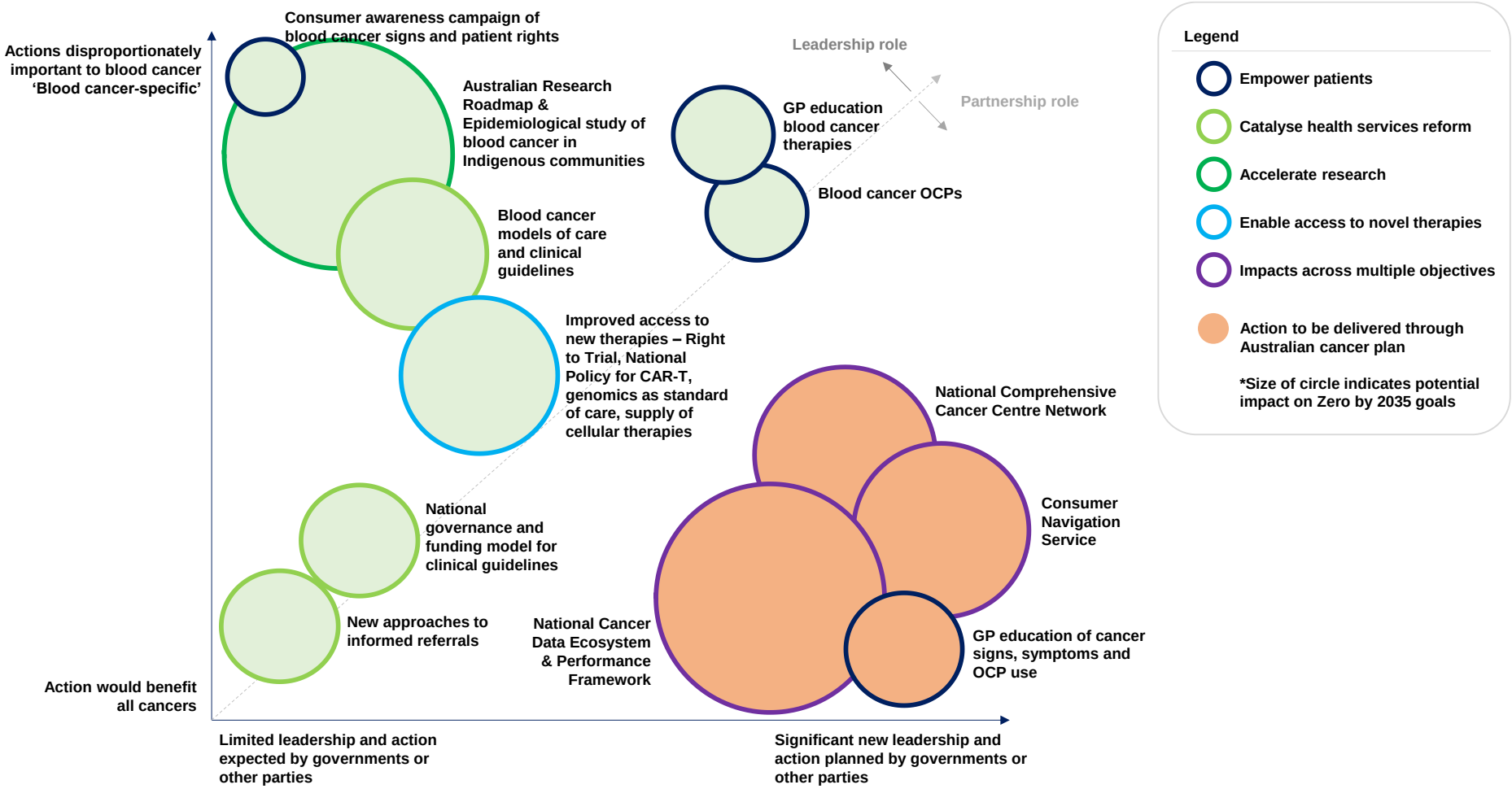


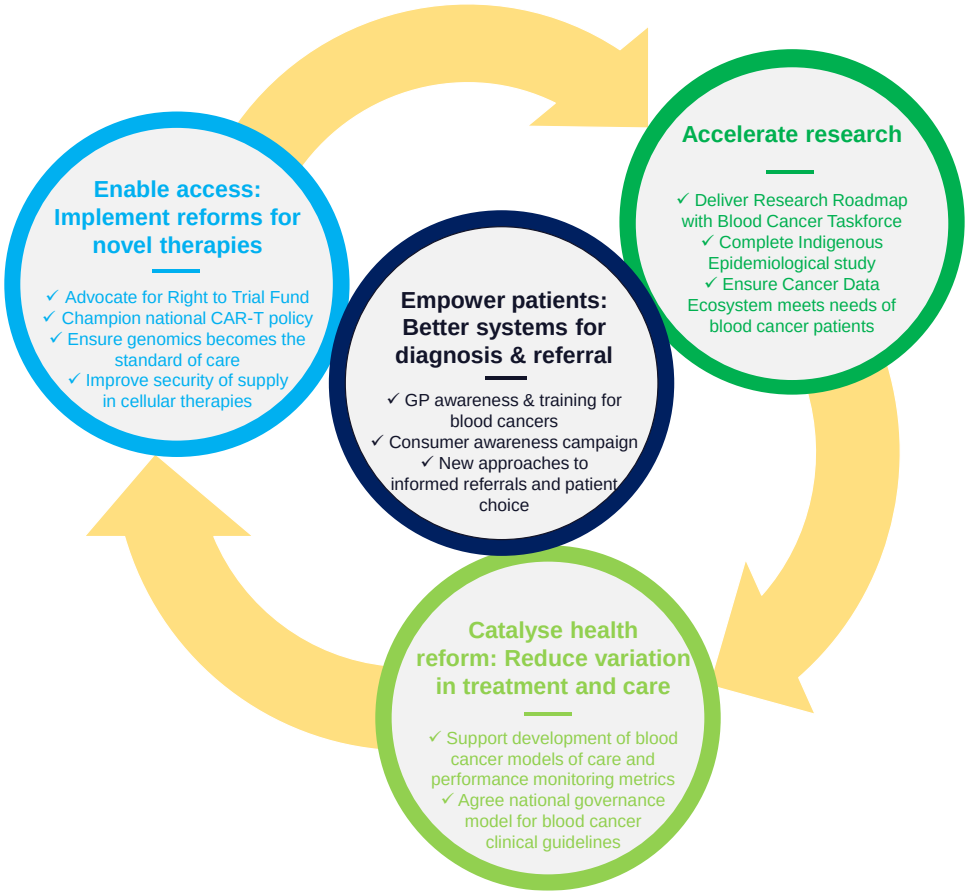
Figure 5: Key actions from the State of the Nation report by strategic objective



Having catalysed the development of an active, system-level policy reform landscape, the Leukaemia Foundation and wider blood cancer community can reorient its resources towards disease-specific 'gaps' not otherwise addressed by broader, system-level policy work. Specifically, over the next horizon, the Leukaemia Foundation and National Blood Cancer Taskforce can work in partnership role with Cancer Australia and Australian governments in the delivery of major multi-lateral reforms, such as the ACP, while taking a leadership role on actions that are 'blood cancer-specific' and not addressed by broader reform efforts (Figure 5, previous page).

Key 'blood cancer-specific' priorities for action in the short term are identified in Figure 6.

Figure 6: Key priorities for action not addressed by broader health and cancer policy reform efforts

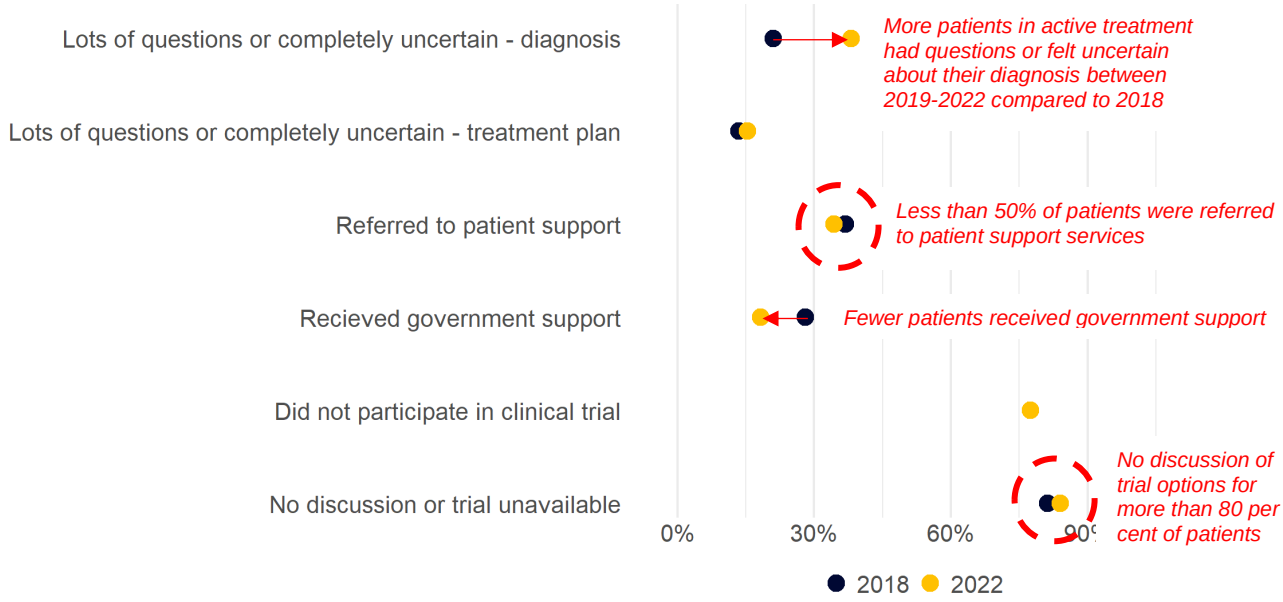


Next horizon priorities to empower patients

Empowering patients and their loved ones to understand their diagnosis, treatment and care options, and to engage effectively with the health system is among the highest priorities for the blood cancer community.

Figure 7 shows that while important enabling infrastructure and actions have been developed since the launch of the first SoTN, very little has improved for people living with blood cancer and against some metrics outcomes have gone backwards. Since the first SoTN, many patients reported feeling more uncertain about their diagnosis and more isolated (because of COVID-19) through their treatment. There was continued low rates of referral to supportive care and poor awareness of clinical trials.

Figure 7: Responses to metrics of treatment and care: comparing 2018 and 2022



Source: People Living with Blood Cancer Survey 2022. See Appendix B.

In light of the expected scope of the ACP, the Leukaemia Foundation, in partnership with the Blood Cancer Taskforce, has the opportunity to deepen its work with Cancer Australia, Australian Governments and the wider blood cancer community to ensure that the major actions of the ACP are implemented in a way that meets the needs of blood cancer patients.

It is proposed that the Leukaemia Foundation, in partnership with the National Blood Cancer Taskforce, works in a partnership role with Australian governments to ensure the implementation of the ACP meets the needs of blood cancer patients. Specific areas of focus and need include the following key areas of action:

- *Consumer navigation and cancer information service* – Actively engage in the co-design of the service and ensure patients are appropriately triaged to blood cancer support services from diagnosis to get the right support throughout their journey
- *National cancer data ecosystem and performance framework* – Actively engage with Cancer Australia in the design of cancer data frameworks, which would integrate clinical, population and Patient Reported Outcome data, and ensure performance metrics of quality and safety meet the needs of people living with blood cancer (e.g., written care plans, referrals to supportive care).

Actions identified by the first SoTN and NAP that are not addressed through other reform efforts and will require blood cancer community leadership include:

- *Education and training of blood cancer signs and symptoms for GPs* – Work with Cancer Australia to develop disease-specific content that supports improved understanding of blood cancers
- *Systems for informed consent and patient choice in referrals* – Work with the blood cancer community to develop an informed consent and patient choice directory of blood cancer services
- *Consumer awareness of blood cancer symptoms* – Implement a campaign to improve community awareness of blood cancers and rights of patients and their loved ones.

Next horizon priorities to catalyse health services reform

Achieving best practice was revealed to be the highest priority for patients from among the four strategic objectives identified to realise the vision of the zero by 2035 goal. This suggests that patients and their loved ones want the health system to work for them – they want to be able to *trust* the health system to deliver the best outcome for them without having to work so hard as their own advocates.

Survey results also showed that patients wanted a performance framework implemented to ensure patients receive quality treatment and care. While performance monitoring is often viewed through a negative lens, it is also an important tool to build trust. Performance reporting also shows where things are going well, so that best practice can be replicated across Australia. The national cancer data performance framework is critical to improve the quality and consistency of adherence to OCPs and to build community confidence in Australia's health system.

Critically, the latest available cancer registry data confirm the number of preventable deaths to be towards the higher end of the range estimated by the first SoTN, with up to 29 per cent of deaths from blood cancer potentially able to be prevented if best practice outcomes were realised nationally.

Many of the key areas of need to catalyse health services reform are planned to be addressed by the ACP. This includes the development of a National Comprehensive Cancer Centre Network and strategies to address workforce challenges, in addition to the actions identified to empower patients (such as GP education, consumer navigation and a cancer data ecosystem). The Leukaemia Foundation and blood cancer community can take a partnership role to ensure the implementation of these ACP initiatives meet the needs of blood cancer patients and help deliver on the zero by 2035 vision.

The Leukaemia Foundation should also continue to work in partnership with the Cancer Council and wider blood cancer community to tackle reforms to prevent financial hardship; this was identified as a lead priority for the Cancer Council over the forward horizon.

Areas of need that have been identified by the first SoTN and NAP that are not going to be addressed through other reform efforts, where the Leukaemia Foundation and blood cancer community have a leadership role to play in national reform efforts, include:

- *Support the development of blood cancer models of care and clinical guidelines for blood cancer* – Build on Cancer Australia's work to develop innovative models of care and performance reporting within the National Comprehensive Cancer Network to define models of care and develop additional clinical guidelines for blood cancer in partnership with the Blood Cancer Taskforce
- *Champion a national approach to clinical guidelines governance and funding* – Working with the Blood Cancer Taskforce, and building on the Phase 1 funding for the development of a blood cancer clinical guideline, the Leukaemia Foundation should advocate for an efficient and sustainable, national approach to clinical guidelines development. A potential governance model is proposed in this report based on recent approaches to other chronic conditions.

Next horizon priorities to accelerate research

Research in blood cancers has made tremendous strides in the past 15 years, with the successful development and application of therapies leading to significant improvements in survival. Combined, blood cancers have achieved among the most significant improvement in 5-year survival over the past 34 years of any cancer group. Moreover, Australia's blood cancer community have been very significant contributors to these improvements, with citation analysis showing the research community are world leaders in blood cancer research.

However, while significant gains have been made, addressing projected mortality and morbidity from blood cancers requires increased and sustained investment in research. While up to a third of blood cancers may be reduced through improved adherence to clinical best practice, the balance requires new discovery. Research is therefore critical to achieving the vision set out in the NAP for zero lives to be lost to blood cancer by 2035.

A Research Roadmap is currently under development with funding committed by the Leukaemia Foundation. The aim of the Research Roadmap is to map out a 10-year plan to develop an ecosystem that accelerates breakthrough blood cancer research in Australia.

The draft ACP has proposed the development of a national cancer data ecosystem; this presents an opportunity for addressing systematic data issues that limit opportunities to make new breakthroughs in understanding disease biology and treatment. The Leukaemia Foundation and blood cancer community can support the development of this national cancer data ecosystem to ensure it enables breakthroughs in research as well as improving patient experience and health service delivery.

The Leukaemia Foundation has also funded a major epidemiological study of blood cancers in Aboriginal and Torres Strait Islander communities, which will address significant data gaps and support improved service delivery to these communities.

Thus, over the short term, the Leukaemia Foundation and blood cancer community have a leadership role to play in:

- Working with the Blood Cancer Taskforce to deliver the Research Roadmap
- Delivering the epidemiological study of blood cancer in Aboriginal and Torres Strait Islander Australian communities
- Ensuring the national cancer data ecosystem is designed and delivered in a way that supports the realisation of the zero by 2035 vision.

Over the longer term, the Leukaemia Foundation, Blood Cancer Taskforce, the blood cancer community, and state and federal governments (including the National Health and Medical Research Council), should partner to fund the implementation of the Research Roadmap.

Next horizon priorities to enable access to novel and specialised therapies

Blood cancer research continues to deliver significant innovation across a range of treatments, including novel immunotherapies, targeted therapies and breakthrough cellular therapies, which are enabling remarkable improvements in survival. Blood cancer survival rates are improving in direct response to research and access to new therapies (Figure 2).

Evidence suggests, however, that there remains inconsistent access to novel and specialised therapies across Australia. This contributes to potentially preventable loss of life, poor quality of life, and inequitable outcomes across patient groups.

Since the launch of the first SoTN in 2019, there has been a significantly improved understanding of the barriers to access and an acknowledgement by government that reforms are needed to address these barriers. While reform reviews are still ongoing, the recognition of the need for reform and catalyst of associated review efforts is a major achievement (Figure 8).

Figure 8: Improve access to novel therapies: priorities for the next horizon

The Leukaemia Foundation, working in partnership with the wider blood cancer community, now has a major opportunity to build on these reform initiatives to see generational, step-change improvements in access arrangements for novel therapies in blood cancer.

As a therapeutic area that is leading the field in the development of novel diagnostics and treatments, the Leukaemia Foundation and Blood Cancer Taskforce have a strong leadership role to play in partnership with federal and state governments to provide the patient and clinical expertise needed to address the barriers that have now been widely and repeatedly acknowledged by government. The Leukaemia Foundation, working in partnership with the Blood Cancer Taskforce, should proactively work with government to:

- Support the establishment of an independent Centre for Precision Medicines and Rare Diseases (CPMRD) as recommended by the recent Parliamentary Inquiry (Zimmerman report)
- Support the Health Technologies Assessment (HTA) review in its development of updated guidance on levels of evidence, additional regulatory and reimbursement pathways, and a revised approach to funding using coverage with evidence development (or managed entry access)
- Serve as an expert reference group on clinically important diagnostics and therapies in blood cancer as part of the horizon scanning to be undertaken by the CPMRD
- Support the establishment of a Right to Trial Fund, as recommended by the House of Representatives Standing Committee on Health, Aged Care and Sport's Inquiry into approval processes for new drugs and novel medical technologies in Australia led by the Hon Trent Zimmerman (the Zimmerman Report), with blood cancers serving as a pilot for the development of the fund over the next two years
- Support the development of a national approach to CAR T-cell services
- Support the development of genomic testing as the standard of care in blood cancer.

Conclusion

This report aims to improve outcomes for people living with blood cancer and is intended to support the whole blood cancer community to continue to work together towards zero lives lost to blood cancer. In focusing on these key areas of action alongside wider health system reforms and working in partnership with the Blood Cancer Taskforce, the blood cancer community has many opportunities to build on its work to date to improve outcomes for people living with blood cancer on the journey to zero lives lost by 2035.

Chapter 1

Understanding Blood Cancers in Australia today: incidence, impact, technology advances and policy reform

Launched in early 2019, the State of the Nation revealed the huge impact of blood cancers on Australian communities, showing blood cancers to be among the most fatal and costly conditions affecting Australians.

Today, blood cancers remain a major priority for Australian communities. Improvements in cancer registry reporting have revealed that historically blood cancers have been under-reported; more accurate data releases have increased the count of people diagnosed with a blood cancer to more than 19,400 people each year, making it one of the most frequently diagnosed cancers in Australia.

The effect of blood cancer can extend for a lifetime, impacting survivors of all ages, their loved ones, and the wider community, including from financial impacts as blood cancers remain among the most costly cancers to treat. In addition, blood cancers demonstrate persistently high rates of potentially preventable mortality, particularly within regional and remote Australians.

Since the launch of the State of the Nation, Australian patients have navigated the impact of the COVID-19 pandemic and experienced the arrival of CAR T-cell therapies in mainstream clinical practice. The Australian policy landscape has also evolved markedly with major system-level reforms underway to improve patient experience and access to treatment.

In light of the active policy reform environment, as well as the impact of COVID-19 and some significant technology advances in clinical practice, it is timely to take stock of progress to date and review the current landscape for blood cancers in order to identify key priorities for the next implementation horizon of the zero by 2035 strategy

1.1 Incidence and mortality of blood cancer in 2022: a major priority for Australian communities

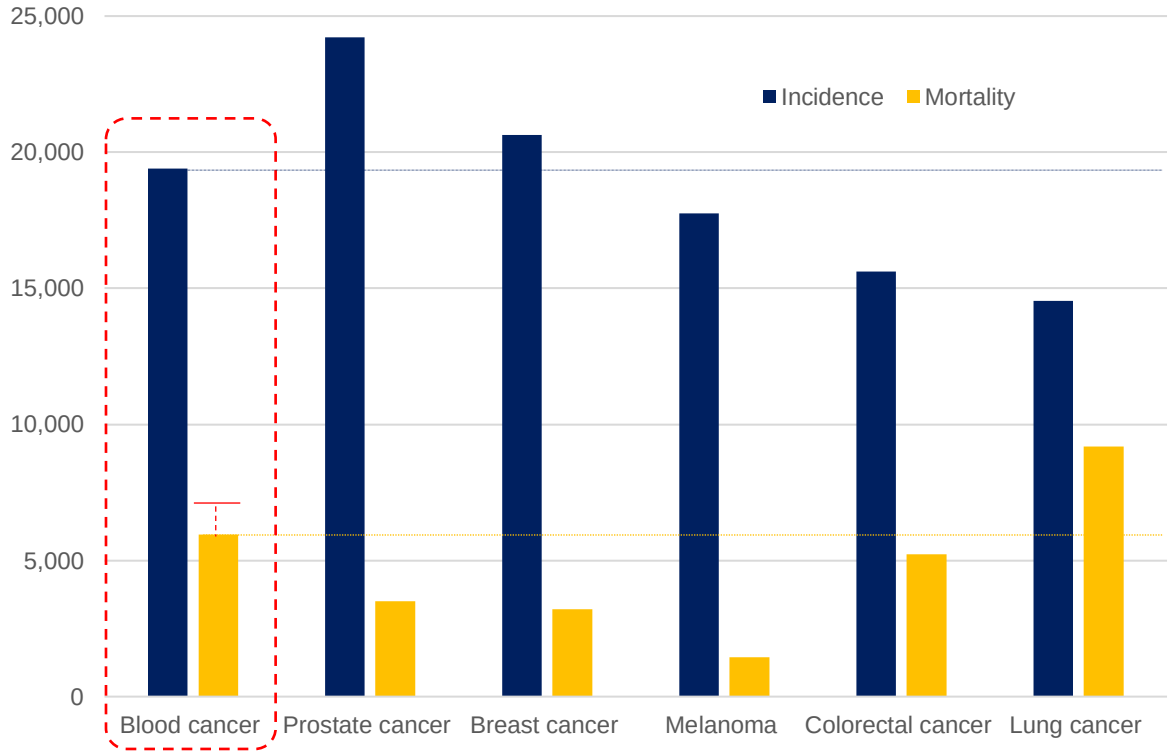
With more than 19,400 people expected to be diagnosed in 2022, blood cancer is among the most commonly diagnosed cancer in Australia today, compared with expected incidence in 2022 of approximately 24,000 for prostate cancer, 20,600 for breast cancer, 17,700 for melanoma, 15,600 for colorectal cancer and 14,500 for lung cancer (Figure 1.1).

Since 2018, the understanding of the incidence of blood cancers has improved. As noted in the State of the Nation (SoTN), it was estimated at that time that approximately 20 per cent of pathology reports for cancer are not submitted by laboratories, and that at least 10 per cent of pathology reports are discarded because they do not meet the criteria for cancer

reporting.[1] In developing the National Strategic Action Plan for Blood Cancer (NAP), Working Group experts similarly reviewed data in regional NSW settings which indicated that reported incidence of blood cancers is lower than true blood cancer volumes.[2] In the latter half of 2019, the Victorian Cancer Registry implemented a Victorian Cancer Registry E-Path Project which saw the incidence of blood cancers reported in Victoria increase. Critically, Victoria's cancer registry is one of the most mature services in Australia, indicating that incidence is likely underestimated nationally; equivalently, national understanding of the incidence of blood cancers remains inadequate to identify the true extent of blood cancers.[3]

Today, the number of people diagnosed with a blood cancer is estimated to be more than 19,400 Australians, a 30 per cent increase over Federal Government estimates in 2018.

Figure 1.1: Incidence and mortality of blood cancer in Australia by sub-type – AIHW 2022 estimates



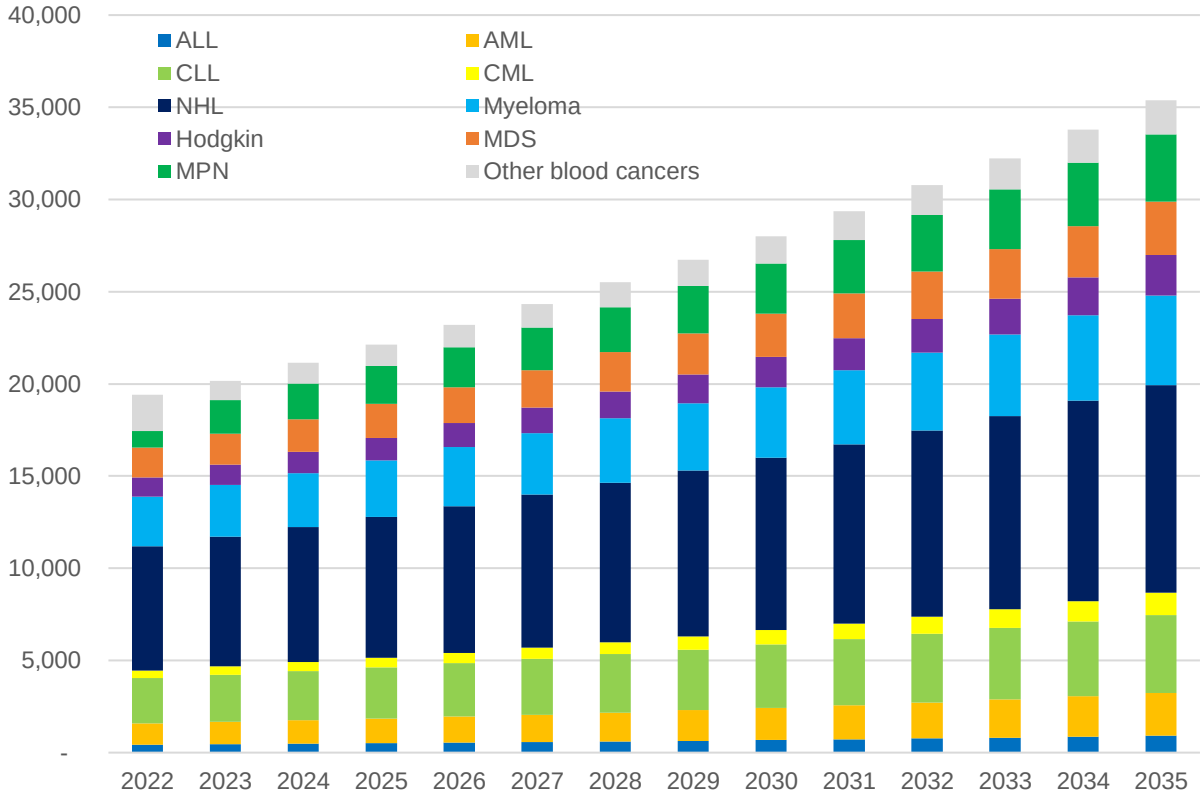
Source: AIHW, 2022, Cancer Data in Australia, incidence projections and mortality count projections (National Mortality Database).

Blood cancer is also among the most frequent causes of death from cancer. Australian Institute of Health and Welfare (AIHW) data estimate approximately 5,900 deaths from blood cancer each year, which is second only to lung cancer in mortality risk among major cancer types. These mortality estimates are based on projections of historical cause of death reported to the National Mortality Database and face similar challenges due to reporting issues. Applying current survival curves by age, sex and region to current incidence and estimated prevalence of blood cancer, would see the number of deaths from blood cancer rise to 6,600 Australians each year.

[1] Cancer Council Victoria, 2019, Victorian Cancer Registry E-Path Project, accessed at: https://www.cancervic.org.au/research/registry-statistics/e-path.
[2] Leukaemia Foundation, 2020, National Action Plan for Blood Cancer, Literature Review, accessed at: https://www.leukaemia.org.au/national-action-plan/.
[3] This may have implications for service delivery, e.g., resourcing decisions.

Cancer registry data also show the incidence of blood cancer is increasing. By 2035, the number of people expected to be diagnosed with blood cancer is projected to rise to more than 35,000 people per annum (Figure 1.2).

Figure 1.2: Incidence of blood cancer in Australia by sub-type – 2035 projections



Source: Insight Economics projections (2022), based on State Cancer Registry and AIHW data by age and gender by blood cancer subtype. See Appendix A.

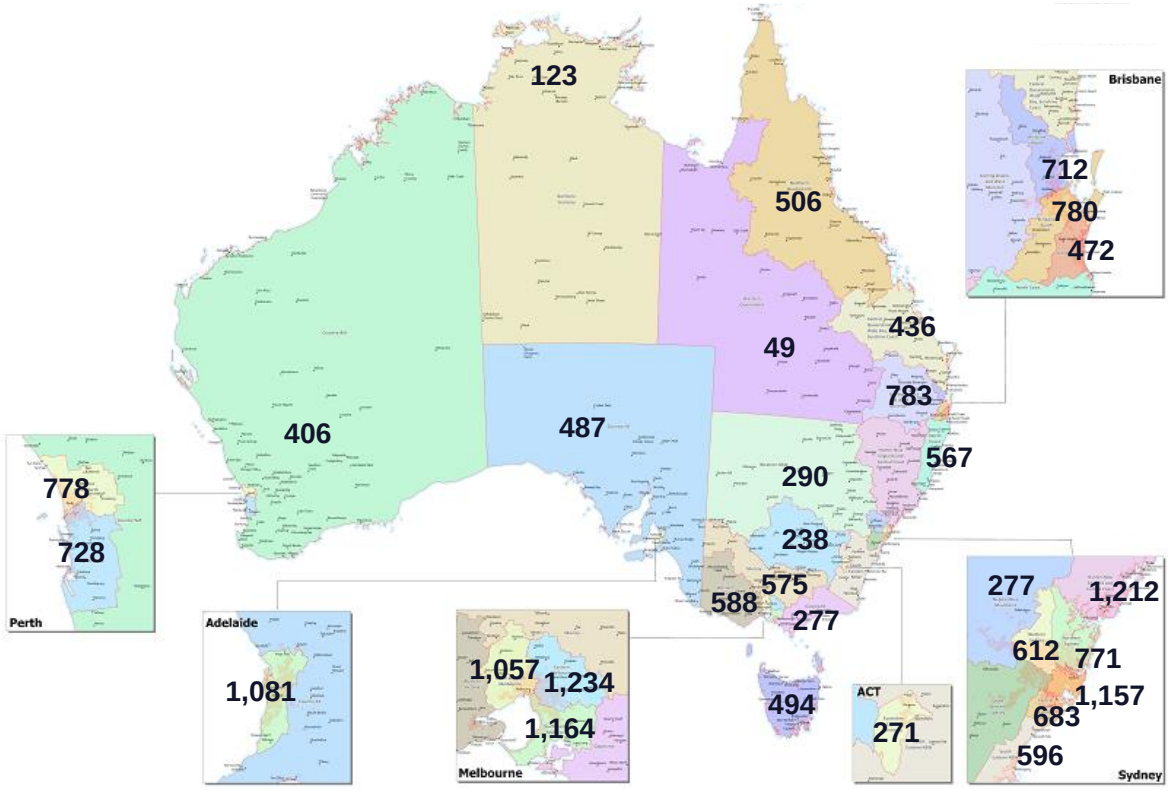
Critically, blood cancers impact people of all ages; based on projections of the incidence of blood cancer between 2022 and 2035:

- More than 7,000 children will be diagnosed with blood cancer; the major sub-types for children include ALL, AML, Non-Hodgkin lymphoma and Hodgkin lymphoma
- More than 6,000 adolescents and young adults (persons aged 15-25 years old) are expected to be diagnosed with broadly the same mix of sub-types as the paediatric cohort
- More than 108,000 adults between the ages of 25 and 65 will be diagnosed, and approximately half of these diagnoses will be for some form of Non-Hodgkin lymphoma

The balance of people diagnosed (more than 250,000 Australians) are expected to be aged over 65 years old, and the mix of blood cancer sub-types shifts towards an increasing incidence of myeloma, CLL, MDS and Non-Hodgkin lymphoma.

Geographically, roughly 59 per cent of people diagnosed with blood cancer in 2022 will be living in a metropolitan area and 41 per cent will be living in a regional or remote area (Figure 1.3).[4]

Figure 1.3: Geographic distribution of blood cancer incidence in 2022 by Primary Health Network (PHNs)



Source: Insight Economics (2022) analysis of state cancer registry and AIHW data.

1.2 Survival has improved, but the costs and preventable mortality of blood cancer remains high

Data show that blood cancer also remains among the most costly conditions to treat, with continued variation in clinical practice and patient outcomes.

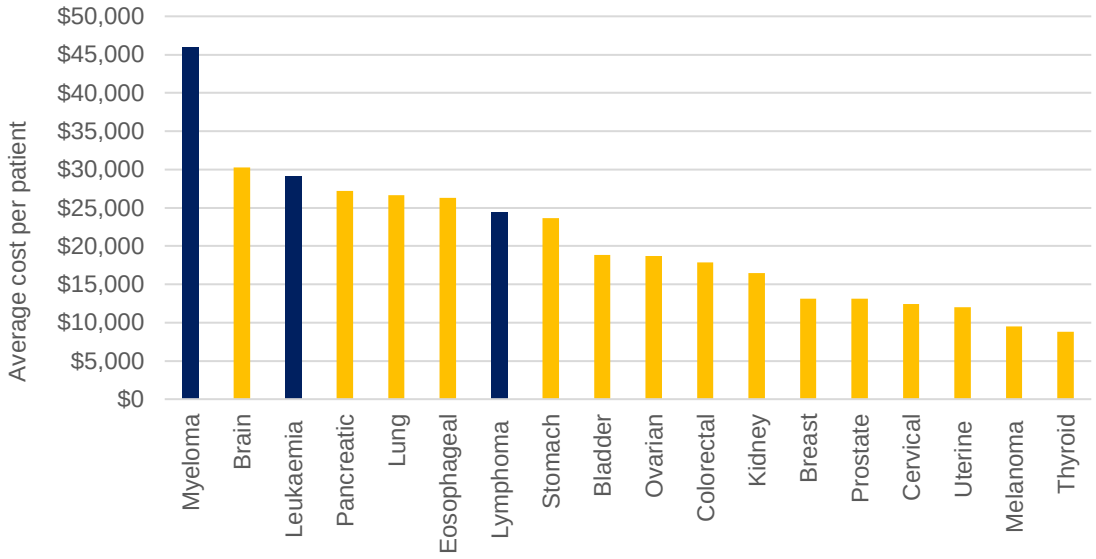
Health system costs of treating blood cancer

Blood cancers remain among the most costly cancers to treat; research published in 2022 found myeloma and leukaemia account for two of the top three most expensive cancers to treat due to high hospitalisation and pharmaceutical costs (Figure 1.4).[5]

[4] Demographic profiles sourced from the AIHW indicates 66 per cent of older people live in major cities (metropolitan areas). See: AIHW, 2021, Older Australians living in rural and remote communities, accessed at: https://www.aihw.gov.au/reports/older-people/older-australia-at-a-glance/contents/diverse-groups-of-older-australians/regional-remote-communities.

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

Figure 1.4: Average cost per patient to treat blood cancer – benchmarking

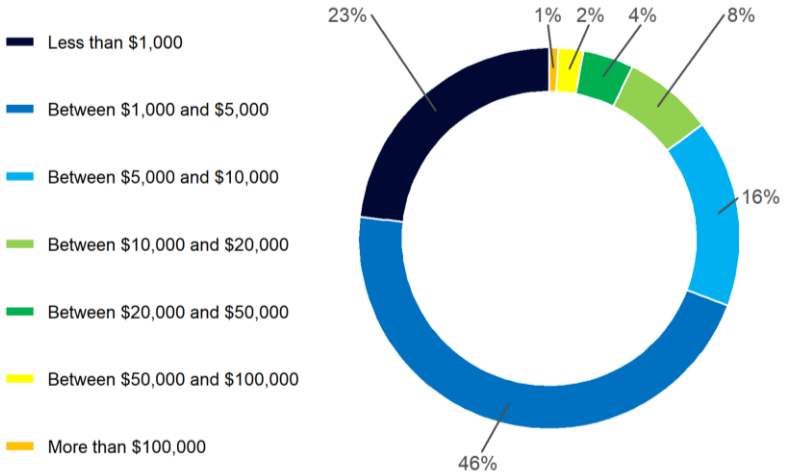


Source: Merollini, K.M.D., Gordon L.G., Ho, Y.M., et al., 2022, Cancer Survivors' Long-Term Health Service Costs in Queensland, Australia: Results of a Population-Level Data Linkage Study (Cos-Q), Int J Environ Res Public Health, 19(15), 9473, doi: 10.3390/ijerph19159473.

Financial costs faced by patients and their loved ones

Blood cancer patients also face high out-of-pocket costs. In the Survey of People Living with Blood Cancer (see Appendix B), of the 43% of patients diagnosed since 2018 who reported out of pocket costs, more than one in three reported incurring more than $A5,000 in cost, while approximately 15 per cent of people incurring out-of-pocket costs experienced costs in excess of $10,000 (Figure 1.5).

Figure 1.5: Out of pocket costs for patients



Source: People Living with Blood Cancer Survey 2022. See Appendix B.

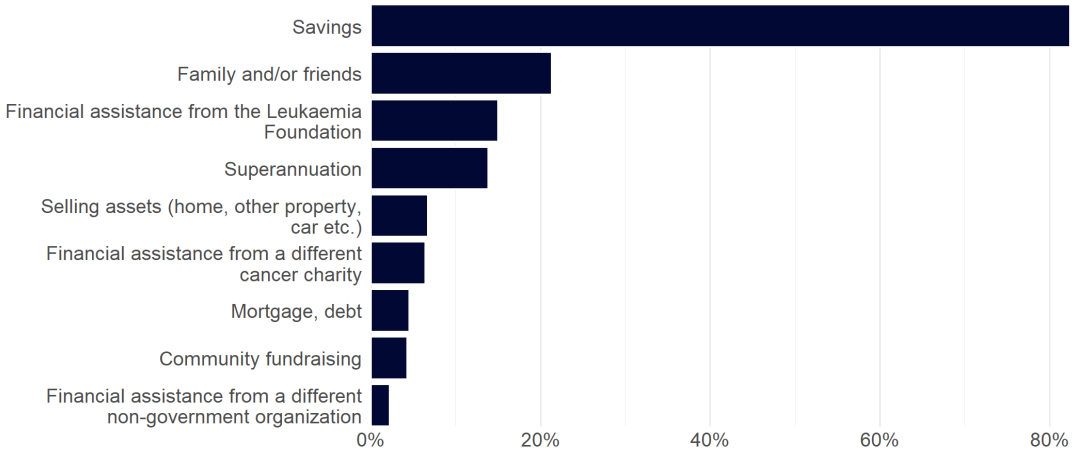
To put these costs in perspective, a 2021 study estimated that, on average, over the 2011-2015 period, patients with a cancer diagnosis paid AU$2462 in out-of-pocket expenses in their first year of treatment.[6]

[6] Rodriguez-Acevedo, A.J., Chan, R.J., Olsen, C.M., et al., 2021, Out-of-pocket medical expenses compared across five years for patients with one of five common cancers in Australia. BMC Cancer 21, 1055, doi: 10.1186/s12885-021-08756-x.

Out of pocket costs increase for patients living in regional and remote areas, who often need to relocate for treatment, and for patients diagnosed with myeloma and leukaemia, who face longer hospital stays and pharmaceutical costs. Respondents to the 2022 Survey of People Living with Blood Cancer indicated frequent out of pocket costs, with 43 per cent of respondents experiencing out of pocket costs and, of those who experience out of pocket costs, 77 per cent experiencing costs more than $1,000 cumulative and 15 per cent experiencing costs in excess of $10,000.

Of those who experienced out of pocket costs, over 80 per cent funded these (at least in part) with savings and seven per cent of had to sell assets to cover these costs (Figure 1.6).

Figure 1.6: How did you cover out of pocket costs?

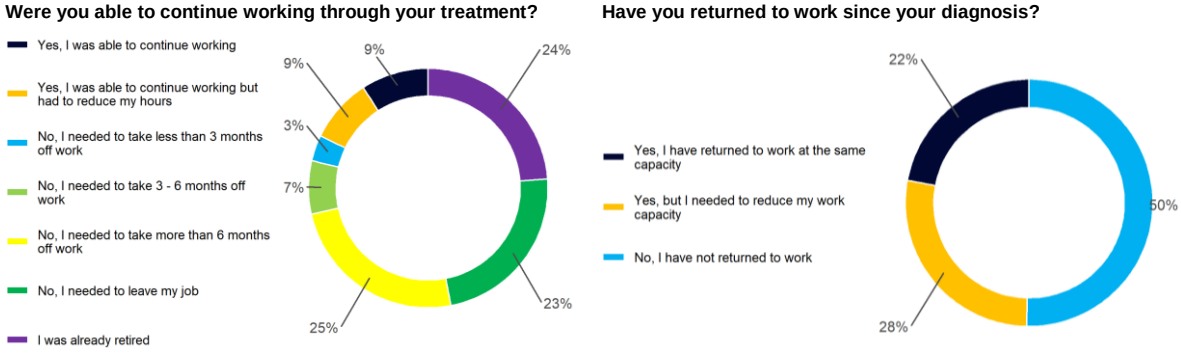


Source: People Living with Blood Cancer Survey 2022. See Appendix B.

Reduced sources of income for patients and carers due to inability to work during treatment and reduced capacity to work following treatment exacerbates hardship:

- Thirty-two per cent of respondents reported having to take over three months off work and 23 per cent reported having to leave their job throughout treatment; 50 per cent of respondents indicated that they have not yet returned to work following treatment, and of those who have returned to work, 44 per cent have been able to return to work at the same capacity (Figure 1.7)
- Eighteen per cent of respondents reported that their carer had to take over three months off work and nine per cent reported that their carer had to leave their job throughout treatment.

Figure 1.7: Ability to work during and return to work following treatment



Note: Respondents who answered with 'not applicable' have been filtered out. Source: People Living with Blood Cancer Survey 2022. See Appendix B.

Figure 1.8 provides a sample of patient comments regarding the financial impact of blood cancers. Examples of financial costs faced by patients include:

- Travel and accommodation, with some blood cancer patients having to move interstate or intra-state to receive treatment
- Unsubsidised diagnostic (entirely or partially)
- Unsubsidised treatments (entirely or partially)
- Allied health and other specialist appointments
- Management of symptoms and side effects
- Cost of medications.

The risks from financial hardship due to the significant, cumulative impacts of treatment for blood cancers are well documented in literature. For example, two recent studies assessing the unmet needs of blood cancer survivors and their carers across Australia showed that patients and carers with multiple high unmet needs[7] were more likely to experience:

- Difficulties paying bills due to cancer
- Use up savings
- Have trouble meeting day to day expenses.[8]

While the association of survival outcomes and unmet needs was not explicitly evaluated by these Australian studies of blood cancer patients,[9] international studies of blood cancer patients show poorer survival outcomes are associated socioeconomic disadvantage and unmet needs. For example, recent analysis of US' National Cancer Institute Surveillance, Epidemiology, and End Results (SEER) Program data showed that in AML and myeloma, non-biological factors were major determinants of survival (Figure 1.9):[10]

- There was a 10 per cent higher risk of mortality by year 5 for low-income persons compared with persons earning above $46,000 per year in 2018, or 23 per cent higher risk of mortality by year 5 between the most disadvantaged quintile of the population compared to the least disadvantaged
- There is a 17-22 per cent higher risk of mortality by year 5 for uninsured persons or persons with limited public insurance coverage (Medicaid or Medicare) compared with persons with insurance
- Divorced or widowed persons were 18 per cent and 33 per cent higher risk of mortality by year 5 compared with married persons.

[7] >7 unmet needs for patients and >6 unmet needs for carers

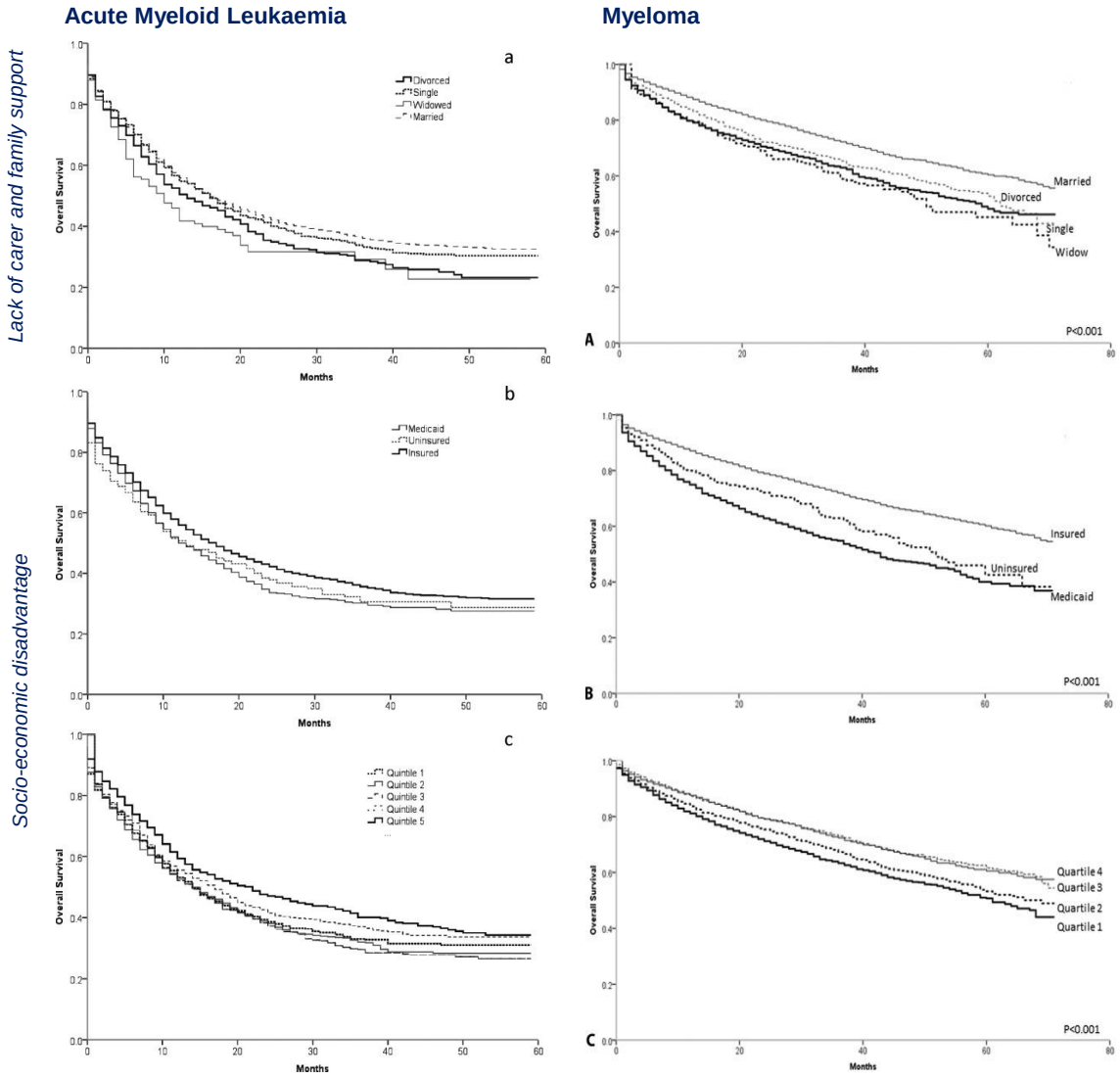
[8] Hall, A., Este, C., Tzelepis, et al., 2014, Factors associated with Haematological cancer survivors experiencing a high level of unmet need across multiple items of supportive care: a cross-sectional survey study, Support Care Cancer, 22:2899-2909, doi: 10.1007/s00520-014-2264-6; Lynagh, M.C., Williamson, A., Bradstock, K., et al., 2017, A national study of the unmet needs of support persons of haematological cancer survivors in rural and urban areas of Australia, Supportive Care in Cancer, 26, 1967-1977, doi: 10.1007/s00520-017-4039-3.

[9] It is noted, however, that Table 3 of the study shows "haematological cancer survivors reporting a disease recurrence compared to those not reporting a recurrence, had higher median domain scores for the Financial Concerns, Access and Continuity of Care and Emotional Health domains." doi: 10.1007/s00520-014-2264-6.

[10] Master, S, Munker, R, Zhenzhen, S., Mills, G, and Shi, R, 2014, Insurance Status and Other Non-biological Factors Predict Outcomes in Acute Myelogenous Leukemia: Analysis of Data from the National Cancer Database, International Journal of Cancer Research and Treatment; Borate UM, Mineishi S, Costa LJ. Nonbiological factors affecting survival in younger patients with acute myeloid leukemia. Cancer. 2015 Nov 1;121(21):3877-84. doi: 10.1002/cncr.29436: Jamy O, Xavier AC, et al, 2018, Impact of Insurance Status on Survival of Patients Diagnosed with APB in the US, ASH Annual Meeting, San Diego; Costa, LJ, Brill IK, Brown EE, 2016, Impact of martial status, insurance status, income.and race/ethnicity on the survival of younger patients; Costa LJ, Brown EE, 2015, 'Insurance Status, Martial Status, Income but not Race-Ethnicity Affect Outcomes of Younger Patients Diagnosed with Multiple Myeloma in the US', Blood Journal, 126(23): 633.

Figure 1.8: Patient perspectives on costs experienced (Survey of People Living with Blood Cancer, 2022)

Figure 1.9: Non-biological factors to overall survival in AML and myeloma



Source: Costa, L, 2016, Impact of marital status, insurance status, income, and race/ethnicity on the survival of younger patients diagnosed with multiple myeloma in the United States, Cancer, 122(20): 3183-3190, doi: 10.1002/cncr.30183; Borate, UM, et al, 2015; Nonbiological factors affecting survival in younger patients with acute myeloid leukemia, Cancer, 121(21):3877-3884, doi: 10.1002/cncr.29436.

Survival and preventable mortality

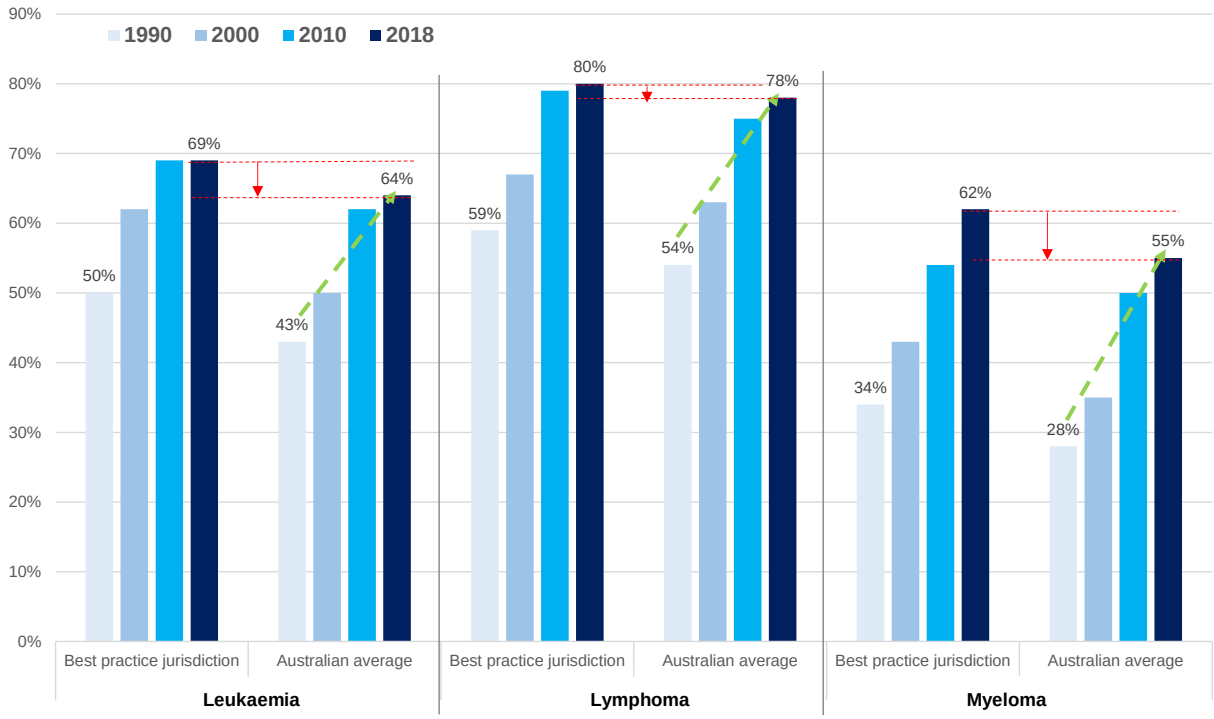
Overall, cancer registry data show survival has improved substantially since the launch of the first SoTN, driven mainly by access to new therapies. At the same time, updated cancer registry data show preventable mortality remains a significant issue for people diagnosed with blood cancer and their families.

The first SoTN estimated that between 13 per cent and 30 per cent of blood cancer deaths may be preventable. To be conservative, the estimate of preventable deaths in the first SoTN was based on the lower bound estimate (13 per cent), which was based on the latest available cancer registry data that time (2011-2015 data). Expert opinion at that time (2018) estimated, however, that the actual figure was potentially much higher: potentially in the order of 30 per cent. Since the launch of the first SoTN new data have been released by state cancer registries for the 2015-2019 period. These latest registry data, which reports outcomes over the 2015-2019 period, confirms the expert opinion was a more accurate assessment of the gap. The latest available cancer registry data show if best practice

outcomes were achieved, potentially up to 29 per cent of blood cancer mortality could be prevented. This underscores the conservative estimates of the first SoTN, and underline the need for reforms to improve consistent access to best practice treatment.

Thus, while there has been significant improvements in survival nationally over the past two decades, there remains significant variation in outcomes depending on where a patient is treated. To illustrate this point, Figure 1.10 shows a historical perspective of 5-year relative survival outcomes for patients nationally and in the state with the best survival outcomes by age, sex and blood cancer sub-type (weighted averages presented). As shown in the Figure, survival has improved substantially on the back of research breakthroughs and improved access to novel therapies for all Australians (the national average), but variations in outcomes persist among Australian jurisdictions such that the national average consistently lags best practice. Eliminating this gap for all age cohorts, gender and region would be expected to reduce projected mortality.

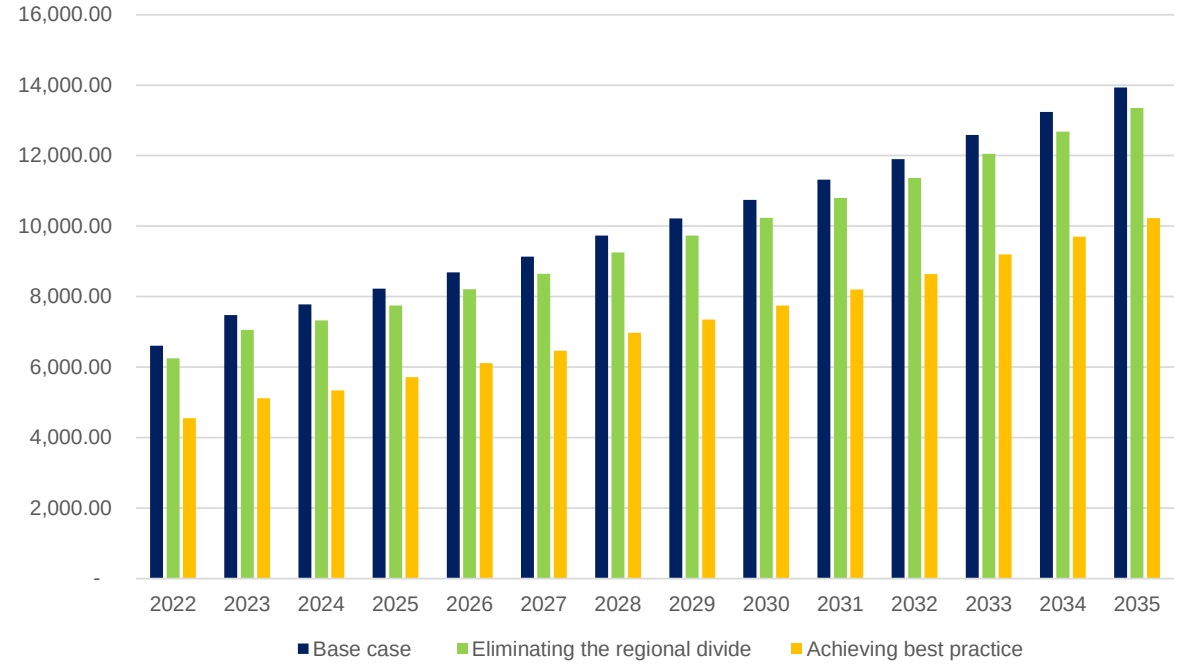
Figure 1.10: 5-year relative survival outcomes by major blood cancer sub-type (1990-2018) – comparing the national average to the best practice jurisdiction



Source: Analysis of Cancer Council data.

When variation in outcomes are eliminated for each cohort, stratified by age, gender, blood cancer sub-type and region based on latest available cancer registry data, this shows that up to 29 per cent of projected blood cancer mortality could be prevented. Over the 2023-2035 (inclusive) horizon, this translates into more than 38,200 preventable deaths or roughly 2,900 deaths per annum on average (Figure 1.11).

Figure 1.11: Mortality projections – benefits from achieving best practice



Source: Analysis of state cancer registry and AIHW data.

1.3 The physical, emotional and social impacts of blood cancer

Many of the challenges faced by blood cancer patients are shared with the broader cancer community (such as physical effects of chemotherapy); however, there are a subset of factors that are driven by the unique features of blood cancer and current practice in Australia.

The first SoTN and NAP identify features of blood cancer which lead to unique or intense lifestyle challenges:[11]

- There is high incidence of certain blood cancers in paediatric, adolescent, and young adult populations; for example, acute lymphoblastic leukaemia (ALL) is the most commonly diagnosed cancer in people aged 0 to 19,12 and aggressive treatments of blood cancer in paediatric, adolescence and young adult populations can have significant and life-long side effects in particular for those individuals who undergo a stem cell transplant.
- Blood cancers are a group of heterogeneous rare or uncommon cancers; there are over 120 discrete blood cancer types and subtypes, including more than 40 unique sub-types of leukaemia, more than 50 unique sub-types of lymphomas, and increasing recognition of a range of myelodysplastic syndromes and myeloproliferative neoplasms
- Blood cancers are spontaneous and unable to be detected through population screening programs like many other major cancers
- For chronic or indolent subtypes, a 'watch and wait' approach to treatment is adopted for months to years

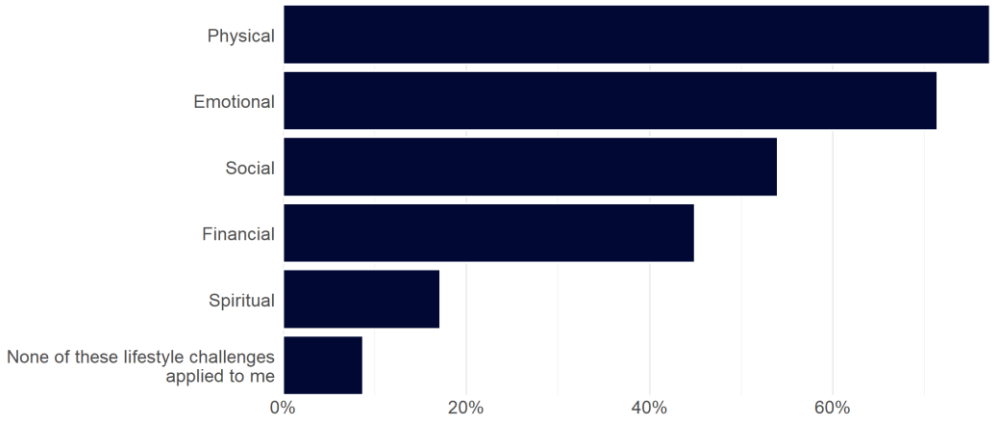
[11] Leukaemia Foundation, 2019, State of the Nation: Blood Cancer in Australia.
[12] AIHW, 2022, Cancer data in Australia, Cancer rankings data visualisation, accessed at: https://www.aihw.gov.au/reports/cancer/cancer-data-in-australia/contents/cancer-rankings-data-visualisation.

- For acute or aggressive subtypes of blood cancer, treatment leads to radical disruptions to patient and family life, often involving long hospital stays and the need to relocate to a capital city to access specialist treatment. In addition, even with new and targeted therapies being developed, many blood cancers are incurable, with treatment (lasting months or years) provided to prolong life or treat symptoms
- Blood cancer patients often deal with multiple instances of remission and relapse, and blood cancers can become refractory to treatment
- Patients are often immunocompromised during active treatment and as survivors.

The physical, emotional, social and financial challenges faced by people diagnosed with blood cancer are among the most significant that can be faced by a family. Respondents to the 2022 Survey of People Living with Blood Cancer highlighted the substantial impact blood cancers have on the lives of Australians:

- 77 per cent of respondents reported physical challenges
- 71 per cent of respondents reported emotional challenges
- 54 per cent of respondents reported social challenges
- 45 per cent of respondents reported financial challenges.

Figure 1.12: Lifestyle impacts of blood cancer



Note: (Q) What lifestyle challenges have you experienced as a result of your blood cancer? (select all that apply). Source: People Living with Blood Cancer Survey 2022. See Appendix B.

Figure 1.13: Patient perspectives on physical and emotional impacts of blood cancer

Source: Survey of People Living with Blood Cancer (2022); stakeholder consultations.

Physical challenges of blood cancer

Physical symptoms and side effects are often consistent with other forms of cancer (such as fatigue). Although patients may live with limited or no symptoms, when they emerge, they can include:

- Extreme **fatigue, infection and bruising**, brought on by low blood counts
  - Shortage of red blood cells (anaemia), which can induce high levels of fatigue
  - Shortage of normal white blood cells (leukopaenia), which can increase the risk of infections such as pneumonia
  - Shortage of blood platelets (thrombocytopenia), which can lead to excess bruising, bleeding, nosebleeds and bleeding gums
- **Pain, numbness and muscle weakness, brought on by bone problems and effects to the central nervous system**:
  - Bone pain, including in back, hips and skull
  - Bone weakness, either all over (osteoporosis) or where there is plasmacytoma
  - Broken bones (fractures), including from minor stress or injury
- **Kidney problems and kidney failure, pain and dehydration** as a result of high blood levels of calcium (hypercalcemia)
- **Heart conditions associated with treatment, eye and skin issues and excess production of other proteins,** such as immunoglobin M (Waldenstrom's Macroglobulinemia, WM).

The chronic nature of some blood cancers means that physical symptoms can persist for long periods of time, reducing quality of life but not leading to presentation to tertiary care. The consequence of often ambiguous long-term symptoms is that patients can suffer challenges such as fatigue for long periods of time without knowing why.

Physical challenges associated with the treatment of blood cancers are often unique, relating to treatment regimes. Stem cell (bone marrow) transplants are one example of a mode of therapy which is relatively unique to blood cancer. Despite being potentially curative, stem cell transplants are associated with serious mortality and morbidity risks. Relatedly, patients receiving transplants are required to spend substantial time in hospital and stay nearby post-transplant.

A further complication arising from blood cancers and treatment is that patients can become immunocompromised, resulting in patients and their loved ones feeling the need to self-isolate. This has been of particular significance to people living with blood cancer during the COVID-19 pandemic.

Emotional and social challenges of blood cancer

People living with blood cancer and their loved ones can suffer significant emotional outcomes, including clinically significant anxiety and depression. A systematic review in 2018 found the prevalence of severe depression, minor depression and anxiety to be significantly higher in people living with cancer than the general population, and that people living with blood cancers reported among the highest levels of anxiety, with clinically significant anxiety persisting for up to 10 years.[13] This same study found that the rate of depression and anxiety is highest for patients with unmet needs.[14]

With some indolent or chronic forms of cancer, patients may be required to 'watch and wait' for symptoms or markers to progress before undergoing treatment. This can contribute to mental health challenges for patients, for example, relating to fear of future progression.

The long length of treatment and time away from home also contributes to emotional and social stress. For example, patients may need to relocate to accommodation closer to hospitals and this separation from home, family and social support networks may lead to disruption of patients and their loved ones.

Psychosocial disorders adversely impact patient capacity to cope with disease burden and may reduce patient adherence to recommended treatments leading to more frequent cancer recurrence.[15] Similarly, studies have found that mortality at one-year post-bone marrow transplant is influenced by psychological distress.[16]

Psychological distress is also likely to be higher when disease burden or complications are more severe. For example, in patients who have had a bone marrow transplant, the level of

[13] Pitman, A., Suleman, S., Hyde, N., et al., 2018, Depression and anxiety in patients with cancer, BMJ, doi: 10.1136/bmj.k1415.

[14] >7 unmet needs for patients and >6 unmet needs for carers.

[15] Hall, A., Este, C., Tzelepis, et al., 2014, Factors associated with Haematological cancer survivors experiencing a high level of unmet need across multiple items of supportive care: a cross-sectional survey study, Support Care Cancer, 22:2899-2909, doi: 10.1007/s00520-014-2264-6; Lynagh, M.C., Williamson, A., Bradstock, K., et al., 2017, A national study of the unmet needs of support persons of haematological cancer survivors in rural and urban areas of Australia, Supportive Care in Cancer, 26, 1967-1977, doi: 10.1007/s00520-017-4039-3.

[16] Clinical practice guidelines for the psychosocial care of adults with cancer, prepared by the National Breast Cancer Centre and the National Cancer Control Initiative Funded by the Department of Health and Ageing, 2003, A National Health Priority Area Initiative, accessed at: https://www.canceraustralia.gov.au/sites/default/files/publications/pca-1-clinical-practice-guidelines-for-psychosocial-care-of-adults-with-cancer_504af02682bdf.pdf; Sharpe, M., Walker, J., Holm Hansen, C., et al., 2014, Integrated collaborative care for comorbid major depression in patients with cancer (SMaRT Oncology-2): a multicentre randomised controlled effectiveness trial, The Lancet, 384(9948), 1099-1108, doi: 10.1016/S0140-6736(14)61231-9.

anxiety was found to be significantly higher in those patients who develop grade II-IV graft-versus-host disease (GVHD) compared to those who developed grade O-I GVHD.[17]

1.4 Impact of COVID-19

The first SoTN was published prior to the COVID-19 pandemic, and the NAP was published at the beginning of the COVID-19 pandemic.

Members of the blood cancer community noted (Figure 1.14) and respondents to the 2022 Survey of People Living with Blood Cancer highlighted that COVID-19 and the associated response has:[18]

- Provided a reminder for how vulnerable blood cancer patients are
- Exacerbated social and emotional challenges for patients and their loved ones, including in treatment where restrictions were placed on visitation and in palliative care[19]
- Created confusion including due to inconsistent vaccination policies
- Exacerbated workforce challenges, including due to mandatory isolation
- Contributed to lost income, including potentially due to the patient or their loved ones losing employment because of workplace closures or choosing not to work in order to mitigate the risk of contracting COVID-19,
- Potentially contributed to delays in access to Centrelink payments
- Promoted adoption of new technologies and models of care, including telehealth
- Provided evidence of the capacity of the international policy and research community to work together efficiently to achieve a common goal.

[17] Clinical practice guidelines for the psychosocial care of adults with cancer, prepared by the National Breast Cancer Centre and the National Cancer Control Initiative Funded by the Department of Health and Ageing, 2003, A National Health Priority Area Initiative, accessed at: https://www.canceraustralia.gov.au/sites/default/files/publications/pca-1-clinical-practice-guidelines-for-psychosocial-care-of-adults-with-cancer_504af02682bdf.pdf based on the original report: Gregurek, R., Labar, B., Mrsic, M., et al., 1996, Anxiety as a possible predictor of acute GVHD, Bone Marrow Transplant, 18:585-9.

[18] See also: Zomerdijk, N., Jongenelis, M., Short, C.E., et al., 2021, Prevalence and correlates of psychological distress, unmet supportive care needs, and fear of cancer recurrence among haematological cancer patients during the COVID-19 pandemic, Support Care Cancer, 29, 7755–7764, doi: 10.1007/s00520-021-06369-5; Vijenthira, A., Gong, I.Y., Fox, T.A., et al., 2020, Outcomes of patients with hematologic malignancies and COVID-19: a systematic review and meta-analysis of 3377 patients, Blood, 136(25), 2881–2892; The Guardian, 2021, 'Totally unacceptable': locked down Australians struggling to access Covid payments.

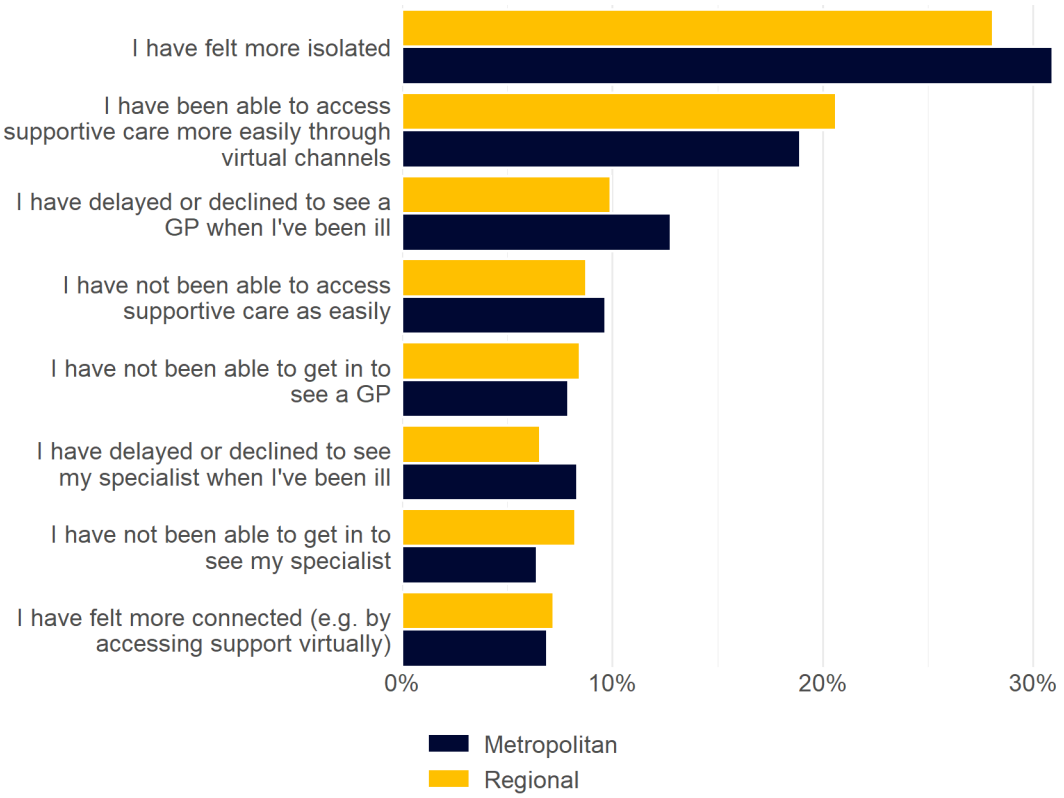
[19] For example, one respondent stated that: "It was extremely difficult due to family not being allowed to visit until 24 hours before her death. I don't want another family to have to go through that with their loved one". Another wrote that "No one should have to do cancer alone and during covid people were expected to. It was unfair".

Figure 1.14: Stakeholder perspectives on the impact of COVID-19

Source: Insight Economics consultation with stakeholders, conducted in late 2022.

There was marked variation in the impact of COVID-19 experienced by respondents to the 2022 Survey of People Living with Blood Cancer. For example, while 20 per cent of respondents felt that it has been easy to access supportive care through virtual channels, nine per cent of patients felt that they have not been able to access supportive care as easily. The most common impact of COVID-19 was that patients felt more isolated (29% of respondents) in treatment and care (Figure 1.15).

Figure 1.15: Impact of COVID-19

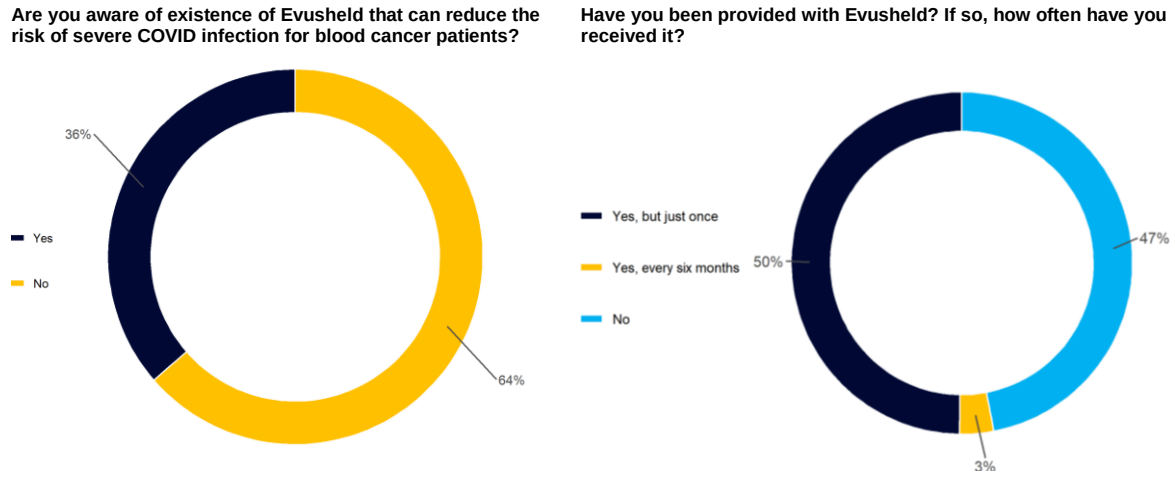


Source: People Living with Blood Cancer Survey 2022. See Appendix B.

Provisionally approved by the Therapeutic Goods Administration (TGA) in February 2022, Evusheld is an injection for prevention of COVID-19 in people with sub-optimal or no protection from COVID-19. Because blood cancer patients often have compromised immune systems, it may reduce the risk of severe infection in blood cancer patients.

Notwithstanding, analysis of survey responses indicates that only 36 per cent of respondents were aware of Evusheld. Among those who were aware of Evulsheld, 53 per cent had received Evusheld (Figure 1.16).

Figure 1.16: Awareness and access to Evusheld



Source: People Living with Blood Cancer Survey 2022. See Appendix B.

Together, only 19 per cent of respondents had been provided with Evulsheld prior to responding to the survey.

1.5 Significant technology advances and policy reform

Other significant changes to the blood cancer landscape since 2018 included significant technology advances, such as the arrival of CAR T-cell therapies, expanded use of telehealth, and the catalyst of a highly active policy reform landscape.

Major technology advances

Two significant changes in technology and service delivery were noted since the launch of the first SoTN, both of which have positively contributed to improvements in patient outcomes:

- *Increased use of telehealth and teletrials* – The pandemic response necessitated the uptake of digital technologies including telehealth. Increased uptake of telehealth marks a major change in how health care is delivered. Teletrials have also emerged as a potential tool for reducing burden faced by regional and remote patients. While virtual care has helped enhance access, members of the blood cancer community suggested that a hybrid model is needed to reflect patient preferences and continued benefits from in-person appointments.
- *The arrival of CAR T-cell therapies* –In the first SoTN, CAR T-cell therapy (involves extraction, isolation, and engineering of T cells to recognise and destroy cancer cells) and immunotherapies (involves activating or suppressing the immune system) were next generation opportunities on the horizon. Members of the blood cancer community observed that these therapies are increasingly available today and are therefore no longer 'opportunities on the horizon'.

Significant policy reforms initiated

The first SoTN saw the Federal Government bring together a National Blood Cancer Taskforce to lead a government-funded, first of its kind National Action Plan for Blood Cancer (i.e., the NAP).

Co-chaired by Chris Tanti, the CEO of the Leukaemia Foundation, and Professor John Seymour, Director Department of Clinical Haematology at the Peter MacCallum Cancer Centre & Royal Melbourne Hospital, the Taskforce has brought together Australia's leading

haematologists, researchers, healthcare professionals, patients and members of the blood cancer community to develop a shared vision for change and plan for action.

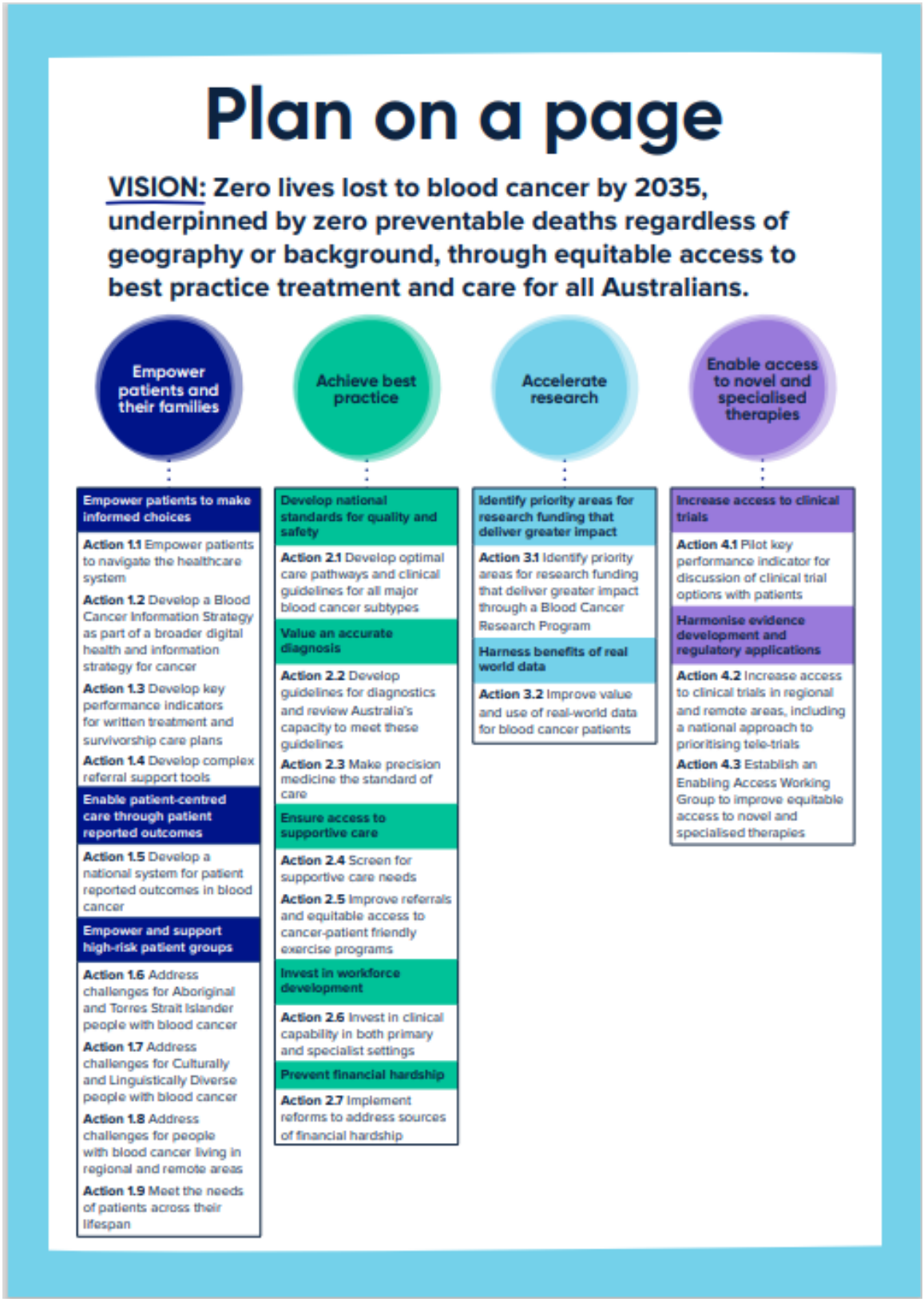
The Taskforce was supported by seven specialist Working Groups, led by members of the Taskforce and drawing on almost 100 external experts to contribute to the preparation of the NAP.

The NAP set out four key goals to realise the vision of zero lives lost to blood cancer by 2035, underpinned by zero preventable deaths, regardless of geography or background, through equitable access to best practice treatment and care for all Australians. The four strategic objectives to realise the vision included:

- Empower patients and their families
- Achieve best practice
- Accelerate research
- Enable access to novel and specialised therapies.

Twenty-one actions were identified for implementation to realise the strategic objectives of the plan and ultimate 2035 vision (Figure 1.17). There was overall strong support for actions and visions set out in the NAP; the NAP was accepted and endorsed by government and 40 other partner organisations.

Figure 1.17: National Action Plan for Blood Cancer Plan on a Page



Source: Leukaemia Foundation, 2020, National Strategic Action Plan for Blood Cancer.

During the COVID-19 pandemic, the Federal Government committed funding for the development of five additional Optimal Care Pathways (OCPs) for blood cancer and initial funding for the development of an approach for clinical guidelines for blood cancer.

Alongside the work on the OCPs and clinical guidelines, and as part of a joint venture agreement with government, the Leukaemia Foundation simultaneously committed to funding the development of a Research Roadmap and a major epidemiological study of blood cancer in Aboriginal and Torres Strait Islander communities.

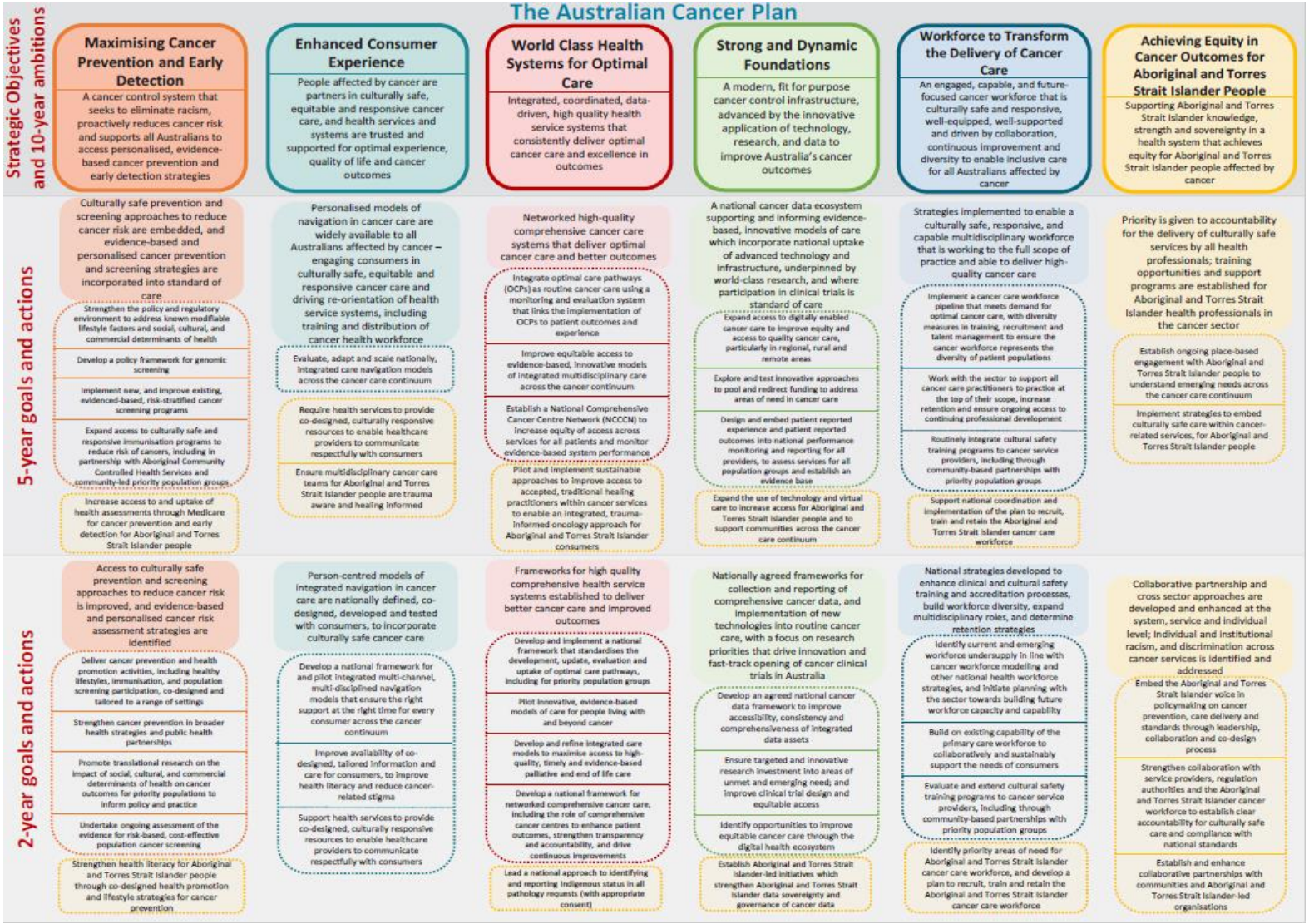
Importantly, many of the ideas championed by the first SoTN and NAP have been adopted within the broader cancer and health policy reform environment (Figure 1.18).

Figure 1.18: Significant policy reform catalysed following the State of the Nation

Source: Summary figure created by Insight Economics

The most significant among these reforms is the development of Australia's first ever Australian Cancer Plan (ACP) by Cancer Australia, which will set out a long-term vision to deliver world class cancer outcomes and experience for all Australians (Figure 1.19).

Figure 1.19: The draft Australian Cancer Plan: strategic objectives, ambitions, goals and actions



Source: Cancer Australia, 2022, Draft Australian Cancer Plan, accessed at: https://engage.australiancancerplan.gov.au/acppc.

Important ideas identified in the first SoTN and NAP are central to the ACP, which noted that many of the ideas put forward by the blood cancer community would be of benefit to all Australians impacted by cancer; the most significant reforms adopted include:

- An integrated Consumer Navigation Service that would improve the availability of disease information to patients and carers and improve the referral of patients to consumer navigation support from diagnosis
- GP education on the signs and symptoms of cancer, to be organised around OCPs
- A national cancer data ecosystem, which would provide the basis for performance reporting by 2028
- The development of a national approach to Patient Reported Outcome measures for cancer
- Development of a National Comprehensive Cancer Center Network and models of care to improve the use of MDTs and regional service delivery.

Cancer Australia is leading the implementation of actions which will benefit all cancers, including blood cancers, and seeking partnerships with patient organisations, including the Leukaemia Foundation, to support implementation consistent with the needs of specific cancers; for example, the implementation of an integrated Consumer Navigation Service will help connect patients with necessary blood cancer support services.

Additionally, multiple government reviews and reform initiatives have commenced, focused on improving access to novel and highly specialised therapies. The House of Representatives Standing Committee on Health, Aged Care and Sport's *Inquiry into approval processes for new drugs and novel medical technologies in Australia,* led by the Hon Trent Zimmerman in 2021 and commonly referred to as the Zimmerman Report, significantly supported the recommendation for a review of access to medicines and the first SoTN recommendations for the creation of a Right to Trial Fund to improve access to clinically important therapies where there is no commercial incentive for evidence development.

1.6 Purpose, method and structure of this report

The first SoTN set a goal that by 2035 there would be zero lives lost to blood cancer. This would be achieved through the removal of barriers to access and addressing inequality in survival outcomes and ensuring that all people living with blood cancer have access to information and best practice treatment and supportive care, resulting in zero preventable deaths by 2035.

Four years on, further to major events including the COVID-19 pandemic, a recent change of Federal Government, and a highly active policy reform environment, it is timely to take stock of progress to date and identify priorities for action. To that end, an update of the People Living with Blood Cancer was undertaken in late 2022, along with modelling, a literature review and stakeholder consultation, to gauge the blood cancer communities' perspectives and better understand progress and areas of need. More than 4,600 people living with blood cancer responded to the consumer survey – a 30 per cent uplift on the previous survey and highlighting the importance of this policy agenda to Australian communities.

Synthesising this evidence, this report presents the key achievements to date against each strategic objective set out in the first SoTN. This report also identifies key areas of need and priorities for the Leukaemia Foundation in light of the wider blood cancer environment:

- Chapter 2 highlights actions to better **empower patients and their loved ones** and proposes key priorities for the next horizon

- Chapter 3 details actions to **catalyse health services reform** and proposes key priorities for the next horizon
- Chapter 4 identifies actions to **accelerate research** and proposes key priorities for the next horizon
- Chapter 5 notes actions to **enable access to novel and specialised therapies** and proposes key priorities for the next horizon
- Chapter 6 brings the ideas from each strategic objective together into a plan for the next 3-5 years for the Leukaemia Foundation.

Chapter 2

Empower People Living with Blood Cancer and their loved ones

Improving survival and wellness starts with the person who is diagnosed with cancer. Supported by their loved ones, the patient knows their history and treatment goals better than anyone else. Getting to an outcome that is best for that person requires their empowerment through information and systems that support them to act.

Putting patients first and at the centre of a plan to improve outcomes for people living with blood cancer was the foundation of the first State of the Nation. The need to empower and support high risk patient groups was emphasised in the National Action Plan.

Since the launch of the State of the Nation in 2019, a number of systemic, multilateral reform initiatives to improve the cancer system have been catalysed, with the development of the first-ever Australian Cancer Plan being chief among these. These reforms are a welcome step and enable the Leukaemia Foundation and its partners to work with Australian governments and the wider blood cancer community to ensure these system-level reforms work in practice for people living with blood cancer.

This chapter briefly reviews the current landscape in which patients and their loved ones engage with the healthcare system, from symptoms through to supportive care, and highlights actions undertaken to date, along with priorities for the next horizon.

2.1 Achievements to date

The first SoTN found that many people find the healthcare system complex and confusing, with substantial barriers to accessing information and services that improve patient outcomes and experience. The report acknowledges that the path to improved survival and wellness begins with the people living with blood cancer. In centering people living with blood cancer and their loved ones, it therefore recommends opt-out referrals to patient support organisations to support consumer navigation, the collection of real-world data on patient outcomes, and the development of systems for complex referral to enable more effective referral pathways.[20]

Building from the evidence and recommendations of the first SoTN, the NAP refined and expanded these recommendations, which were summarised in nine actions within the strategic objective to empower patients; these included:[21]

- Implement a **Consumer Navigation Service**
- Develop a blood cancer **digital health and information strategy**
- Implement a **performance framework against key metrics of quality**

[20] Leukaemia Foundation, 2019, State of the Nation: Blood Cancer in Australia.
[21] Leukaemia Foundation, 2020, National Strategic Action Plan for Blood Cancer.

- Develop **complex referral support tools**
- Develop a national system for **patient reported outcomes** in blood cancer
- Address challenges for **Aboriginal and Torres Strait Islander people**
- Address challenges for **Culturally and Linguistically Diverse people**
- Address challenges for **regional and remote people**
- Address challenges for **people across their lifespan**.

Since the launch of the NAP, major achievements to date include:

- Federal Government has **funded the Blood Cancer Taskforce to develop eleven OCPs,** which improve consumer information and will be the building blocks for cancer system reforms in the implementation of the ACP. Six of these OCPs have already been developed, with the remaining five to be delivered in mid-2023. As part of the development of the OCPs, **companion guides to best-practice cancer care were developed specifically for patients and translated into the eight most commonly spoken languages in Australia**.
- Federal Government has funded the Blood Cancer Taskforce to develop a **clinical guideline for blood cancer**, with the potential for this pilot approach to be replicated and extended to other blood cancer sub-types over time
- Federal Government to develop an integrated **Consumer Navigation Service** and **national consumer cancer information strategy**
- Federal Government to develop a **national cancer data performance framework**, based on markers of quality and safety in the OCPs
- Increased **engagement with GPs to educate them on the signs and symptoms of blood cancer** and the use of the OCPs in guiding further treatment strategies. This includes the Australian Government to fund **future GP education in the signs and symptoms of cancer as part of the implementation of the ACP**, with the implementation plan to specify opportunities for patient support organisations to partner with government around disease-specific challenges
- Federal Government to develop a **national system for Patient Reported Outcomes** in cancer, in order to assess services and establish an evidence base for improving outcomes
- **Improving outcomes for priority populations**, in particular for patients living in regional and remote communities, Aboriginal and Torres Strait Islanders, culturally and linguistically diverse people and people living with blood cancer of different age groups, including children, adolescents and young adults and older Australians.

Optimal care pathways in blood cancer

OCPs are trusted guides that describe what optimal care for a type of cancer should look like. They set out a national standard of high-quality cancer care that all Australians should expect and put the patient at the centre of care decisions.

Covering every step from prevention and early detection through to recovery, living with a chronic disease, or end-of-life-care, the OCPs aim to improve patient outcomes through promoting quality cancer care and ensuring that all people diagnosed with blood cancer receive the best care, irrespective of where they live or receive cancer treatment. OCPs can guide, support and inform increased collaboration, more effective care, improved healthcare provider–patient communication and patient experience.

These OCPs have gone through a national endorsement process, being approved by the Federal Government and all states and territories as best practice in the standard of care for anyone diagnosed with blood cancer in Australia.

Figure 2.1: Optimal Care Pathways for blood cancer in Australia

Source: Optimal Care Pathways, accessed: November 2022, accessed at: https://optimalcarepathways.com.au/ and https://www.cancer.org.au/health-professionals/optimal-cancer-care-pathways.

Publication of the first six OCPs have resulted in the following interactions within the first eight weeks since their launch in August 2022:

- OCPs downloaded over 1,140 times
- Quick Reference Guides downloaded over 460 times
- Guides to Best Cancer Care downloaded over 980 times.

Together, blood cancer patients will benefit from the most complete set of OCPs of any complex cancer group with disease specific information available for all of the major blood cancer sub-types (Figure 2.1). This is a significant improvement since 2018 when only two OCPs had been developed.

National, integrated Consumer Navigation Service in development and consumer information strategy

The first SoTN called for systematic referral of patients to patient support organisations with the goal of helping patients to navigate the health care system. This was echoed and refined in the NAP which called for consumer navigation support. The Federal Government has now committed to the development of a national Consumer Navigation Service; specifically, the draft ACP has set several goals within two years to:

- Develop a national framework for and pilot integrated multi-channel, multi-disciplined navigation models that ensure the right support at the right time for every consumer across the cancer continuum
- Improve availability of co-designed, tailored information and care for consumers, to improve health literacy and reduce cancer-related stigma
- Support health services to provide co-designed, culturally responsive resources to enable healthcare providers to communicate respectfully with consumers.

Within five years, the draft ACP has set goals for the integrated consumer navigation model to be operational:

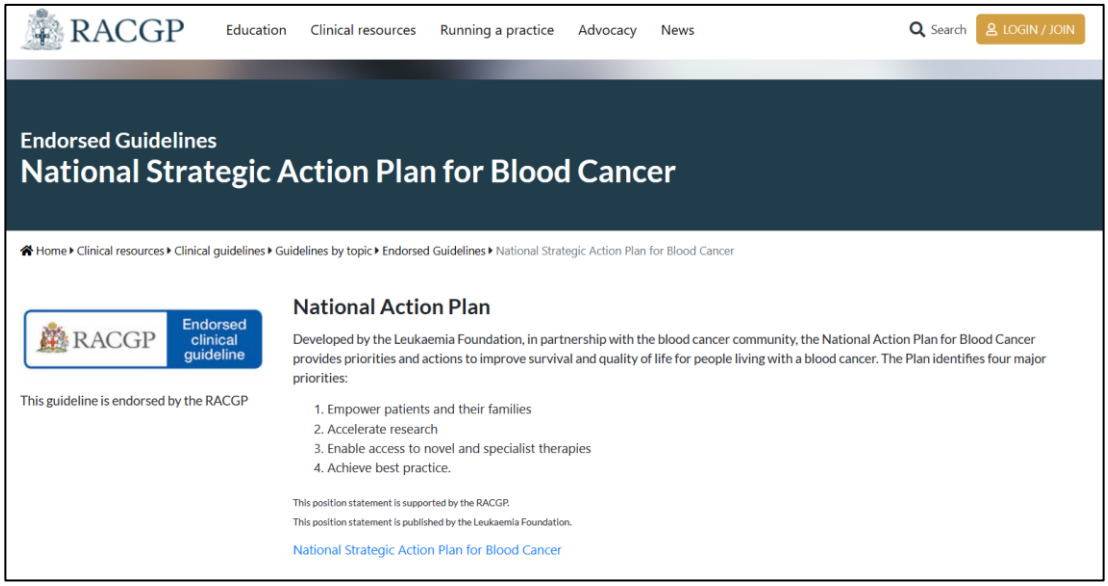
- Evaluate, adapt, and scale nationally integrated care navigation models across the cancer care continuum
- Require health services to provide co-designed, culturally responsive resources to enable healthcare providers to communicate respectfully with consumers.

Funding for GP education into the signs and symptoms of blood cancer

Issues in workforce education and skills availability were identified in the first SoTN across care settings from primary care through to tertiary hospitals. Poor understanding and recognition of blood cancers by GPs being among the most commonly identified issues for patients leading to delays in diagnosis. The lack of GP understanding of blood cancers was reported to lead to inefficiencies in health service delivery, from misdiagnosis, referrals to the wrong specialist, and delays in appropriate treatment.

Since the launch of the first SoTN, the Leukaemia Foundation has worked with the Royal Australian College of General Practitioners (RACGP) to improve awareness and understanding of blood cancers. The RACGP promoted the NAP among GPs and also provided its endorsement (Figure 2.2).

Figure 2.2: RACGP endorsement and promotion of blood cancer awareness among GPs



At the same time, GPs have needed to focus immediate attention on addressing the COVID-19 pandemic, which has limited further engagement with expanding education and skills in other areas.

Looking forward, Cancer Australia is planning additional GP education around the signs and symptoms of cancer, with education to be delivered using the OCPs as the core building

blocks. While education and training will be 'tumour agnostic', meaning that training will apply to the needs of all patients or a specific cohort of patients across different tumour types (e.g., the OCP for Aboriginal and Torres Strait Islander People with cancer), Cancer Australia has indicated that it will partner with appropriate NGOs in the delivery of this education to bring disease-specific considerations to the program. The completion of the five additional OCPs will ensure blood cancer education and training is ready to be delivered as GP education is rolled out.

Poor awareness of the signs and symptoms of blood cancer

Patients are often not aware of the signs and symptoms of blood cancer, and not adequately empowered to push for testing when they observe symptoms, as demonstrated in excerpts from stakeholder consultations highlighted in Figure 2.3 below.

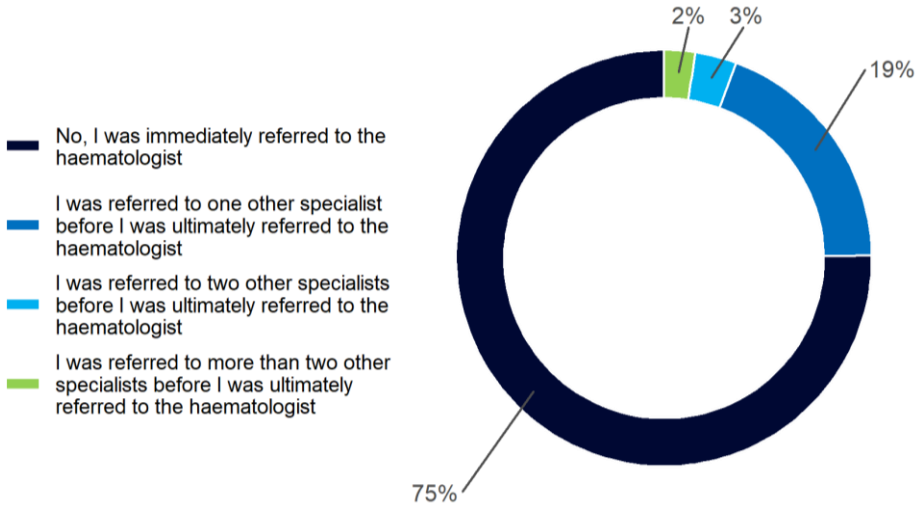
Figure 2.3: Stakeholder perspectives on patient awareness of signs and symptoms of blood cancers

Source: Insight Economics consultation with stakeholders, conducted in late 2022.

Patients need to be empowered to more effectively engage with GPs. Part of this is supporting GPs to better recognise the signs and symptoms so that red flags are more readily identified by consumers and GPs alike.

Poor awareness of the signs and symptoms of blood cancer by GPs and the wider community contribute to delays in diagnosis and detection of blood cancers. Approximately 13 per cent of patients reported it took more than two months from presenting to a GP to obtain a referral to a specialist. Similar to the findings of the first SoTN, the most recent People Living with Blood Cancer survey identified that while most people with blood cancer were immediately referred to a haematologist, a significant number (25%) were referred to two or more specialists before ultimately being referred to a haematologist (Figure 2.4). Positively, the number of patients being referred to two or more other specialists has halved since the previous survey.

Figure 2.4: Were you referred to other specialists before your referral to the haematologist?



Source: People Living with Blood Cancer Survey 2022. See Appendix B.

National system for Patient Reported Outcomes to be developed

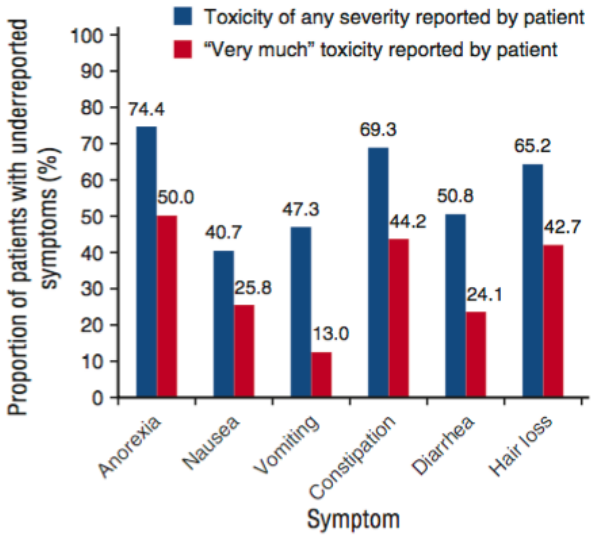
Patient Reported Outcomes (PROs) are patient self-assessments about the status of their health without amendment or interpretation of their response by a clinician or anyone else.

As the treatment of cancer is transforming many cancers from acute, life-threatening diseases to long-term chronic conditions, PROs are increasingly recognised as important tools for symptom and adverse event monitoring to enable better patient care, as well as tools to guide investment in research and regulatory decision making.

Research has found that the symptoms experienced by patients receiving treatment for advanced cancers are undetected by clinicians up to half the time.[22] Clinicians have been found to not only miss a large proportion of patient symptoms (Figure 2.5), but they have also been found to underestimate the impact experienced by patients; potential causes of this include poor verbal communication of symptoms and low attention paid to subjective toxicity, such as where adverse effects are mild.[23] This evidence highlights the important role that PROs have to play in the best practice treatment of patients.

[22] Pakhomov, S.V., Jacobsen S.J., Chute, C.G., Roger, V.L., (2008) Agreement between patient-reported symptoms and their documentation in the medical record, *Am J Manag Care*, 14(8):530-539.
[23] Efficace, F., Gaidano, G., and Lo-Coco, F., 2017, Patient Reported Outcomes in hematology: is it time to focus more on them in clinical trials and hematology practice?, *Blood*, 103(7); Di Maio M, Gallo C, Leighl NB, Piccirillo MC, et al., 2015, Symptomatic toxicities experienced during anticancer treatment: agreement between patient and physician reporting in three randomized trials, J Clin Oncol, 10;33(8):910-5.

Figure 2.5: Proportion of cancer patients with under-reported symptoms



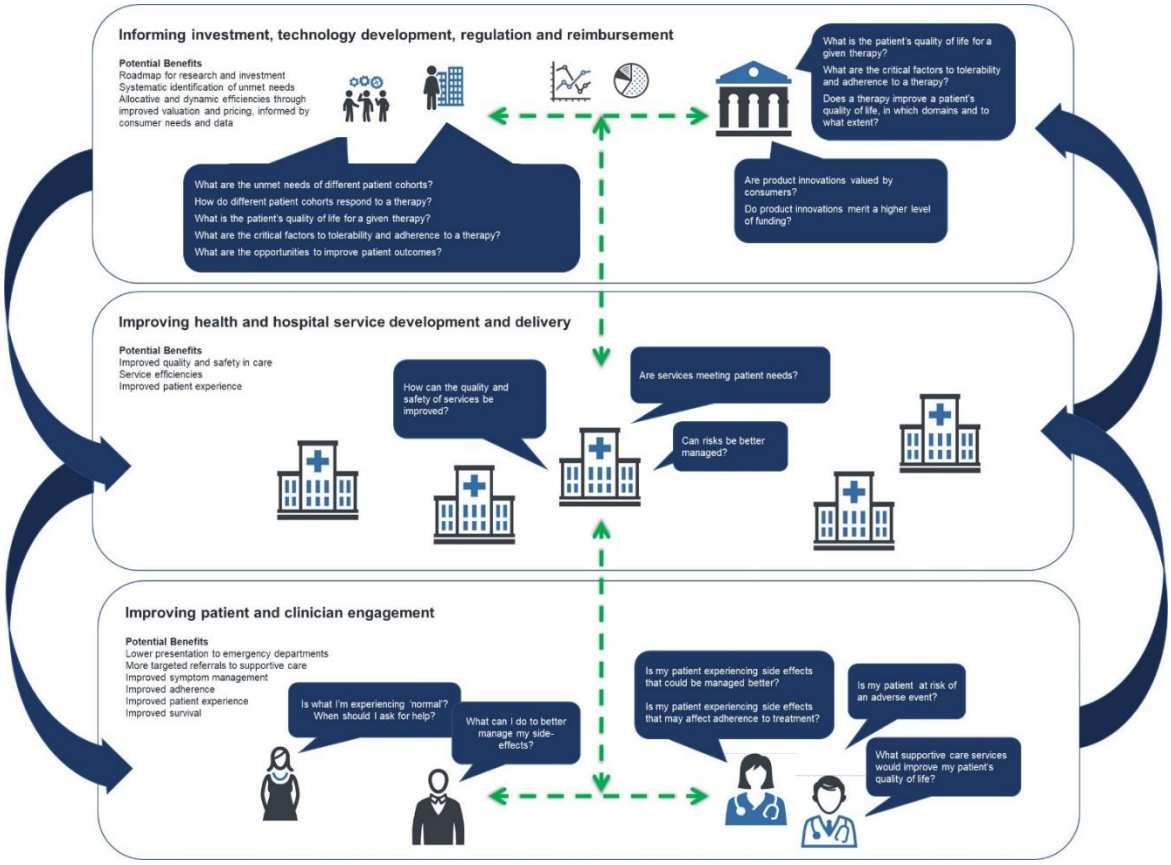
Note: This analysis includes data from 1,090 patients across three randomised trials. Source: Efficace, F., Gaidano, G., and Lo-Coco, F., 2017, Patient Reported Outcomes in hematology: is it time to focus more on them in clinical trials and hematology practice?, Blood, 103(7); based on studies from Di Maio, M., Gallo, C., Leighl, N.B., et al., 2015, Symptomatic toxicities experienced during anticancer treatment: agreement between patient and physician reporting in three randomized trials, J Clin Oncol, 10, 33(8):910-5; Di Maio, M., Basch, E., Bryce, J., et al, 2016, Patient reported outcomes in the evaluation of anticancer treatments, Nat Rev Clin Oncol, 13(5):319-32.

PROs can be used to improve the effectiveness and efficiency of healthcare at three key levels of service delivery and development:

- *Improving patient and clinician engagement* — PROs can improve the effectiveness and efficiency of patient interactions with the healthcare system, by helping the patient to better self-manage their symptoms, or prompting a response by the healthcare practitioner as needed
- *Improving hospital and health service development* — PRO data can be used to monitor, evaluate, benchmark and improve performance and support service development
- *Informing investment, technology development, regulation and reimbursement* —At a macro level, PRO data can be used to inform investment in research by both industry and clinicians, as well as investment and regulatory decisions by governments and other funders.

Figure 2.6 illustrates the three levels at which PROs can influence patient care and decision-making by hospitals, governments, industry and researchers.

Figure 2.6: Patient Reported Outcomes influence care and decision-making



Source: Insight Economics in: Leukaemia Foundation, 2020, National Strategic Action Plan for Blood Cancer.

The NAP presented a detailed evidence case for the development of PROs. This has now been incorporated in the draft ACP, specifically:

- Design and embed patient reported experience and PROs into national performance monitoring and reporting for all providers, to assess services for all population groups and establish an evidence base.
- Expand access to digitally enabled cancer care to improve equity and access to quality cancer care, particularly in regional and remote areas.

Cancer Australia will also establish a governance, national monitoring and evaluation approach for service providers to publish results from patient reported experience and PROs. This aim is to ensure continuous improvements, linked to Action 1 on designing and embedding patient reported experience and PROs into national performance monitoring and reporting for all providers.

Improving outcomes for priority populations

The first SoTN focused on people living in regional and remote areas. The priority population lens was then widened through the NAP to include improved outcomes for Aboriginal and Torres Strait Islanders, culturally and linguistically diverse people and people living with blood cancer of different age groups, including children, adolescents and young adults (AYA) and older Australians.

As part of the Phase 1 implementation of the NAP for Blood Cancer, ,the Leukaemia Foundation, in partnership with the Blood Cancer Taskforce has committed to funding the first national epidemiological study of blood cancer in Aboriginal and Torres Strait Islander

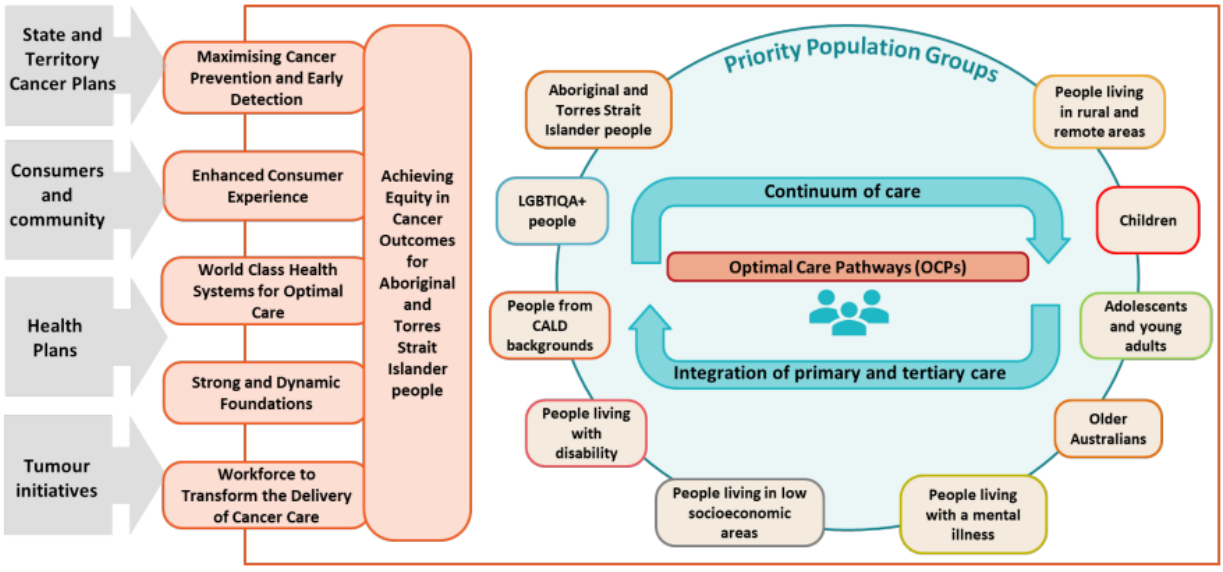
communities. This work will be led by Professor Alex Brown, a leading Aboriginal clinician and Professor of Indigenous Genomics at the Australian National University and Telethon Kids Institute, in partnership with leading research organisations across Australia (Figure 2.7).

Figure 2.7: Research organisations leading epidemiological study of blood cancer in Aboriginal and Torres Strait Islander communities

Source: Leukaemia Foundation

Improving equity of outcomes is also a core feature of the draft ACP. Aboriginal and Torres Strait Islander people, people from culturally and linguistically diverse backgrounds, regional and remote patients and assisting patients across their lifespans have been identified as populations requiring immediate action. Many of these key actions have been incorporated into the draft ACP, which incorporates a priority populations framework for all strategic objectives and actions (Figure 2.8).

Figure 2.8: Priority populations in focus for the draft Australian Cancer Plan



Source: Cancer Australia, 2022, Draft Australian Cancer Plan.

2.2 Key areas of need

As shown in Figure 2.6, there has been significant progress in the development of major, system-level reforms to better empower patients in their diagnosis, treatment and survivorship journey.

At the same time, there is a need to ensure these national reforms, which will be 'tumour agnostic', deliver the changes needed for blood cancer patients. Many patients still report

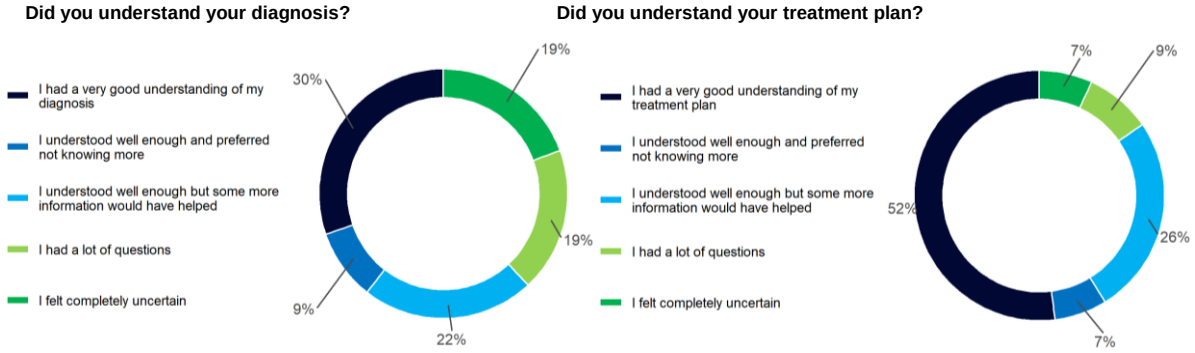
feeling confused by their diagnosis and treatment plan, and report blood cancer-specific difficulties (as discussed in Chapter 1).

Consumer navigation must meet the needs of blood cancer patients

One of the most significant barriers to empowering people living with blood cancer to seek or receive timely and relevant care pathways is the complexity of the healthcare system. Analysis of 4,600 number of responses from the 2022 patient and carer survey (Figure 2.9) indicated that:

- At time of diagnosis, 38 per cent of patients had a lot of questions or felt completely uncertain about their **diagnosis**, while 22 per cent of patients understood well enough but considered that some more information would have helped[24]
- Sixteen per cent of patients had a lot of questions or felt completely uncertain about their **treatment** plan, and 26 per cent understood well enough but considered that some more information would have helped.[25]

Figure 2.9: Patient perspectives – understanding their diagnosis and treatment plan



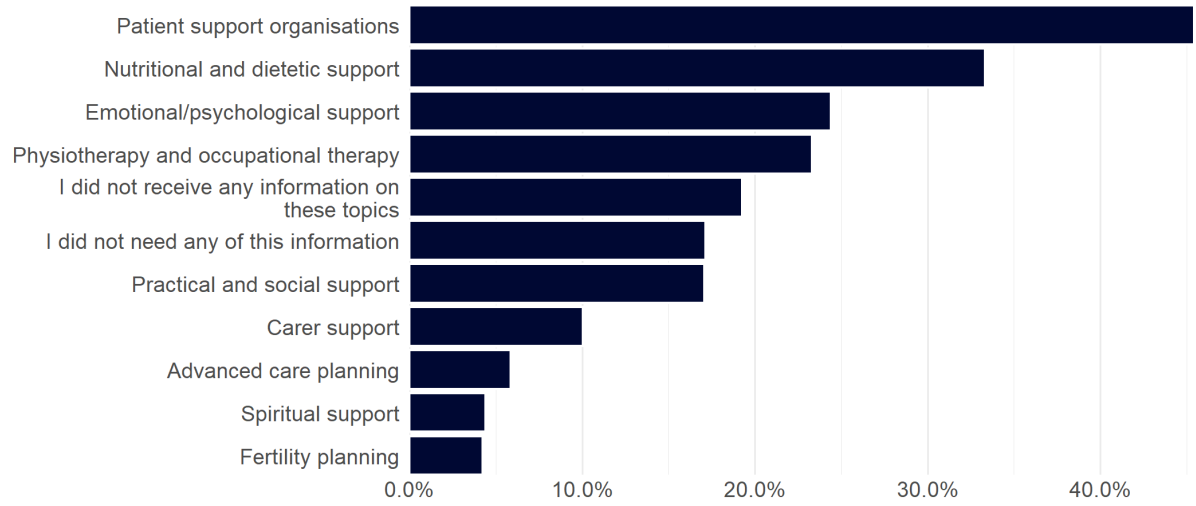
Source: People Living with Blood Cancer Survey 2022. See Appendix B.

Survey data in Figure 2.10 illustrates that a substantial number of patients indicated the desire for more assistance. Areas where patients desire further support include:[26]

- At treatment planning, information about support organisations (28%), emotional and psychological support (27%), physical support (20%), nutrition and dietetic support and practical and social support (19% and 18%, respectively)[27]
- In active treatment, help understanding and managing side effects (31%), help with emotional and psychological support (21%), put in contact with a patient support service (17%), received more help with nutritional and dietetic support (15%).[28]

[24] People Living with Blood Cancer Survey (see Appendix B), Question: At the time when you were diagnosed did you have a good understanding of your diagnosis?
[25] Question: Did you have a good understanding of your treatment plan?
[26] These results are consistent with findings of other Australian surveys. See, for example: Zomerdijk, N., Jongenelis, M., Short, C.E., et al., 2021, Prevalence and correlates of psychological distress, unmet supportive care needs, and fear of cancer recurrence among haematological cancer patients during the COVID-19 pandemic, Support Care Cancer, 29, 7755–7764, doi: 10.1007/s00520-021-06369-5.
[27] Question: What kind of information do you wish you had received during your treatment planning?
[28] Question: In hindsight, would more help in any areas have helped you during active treatment?

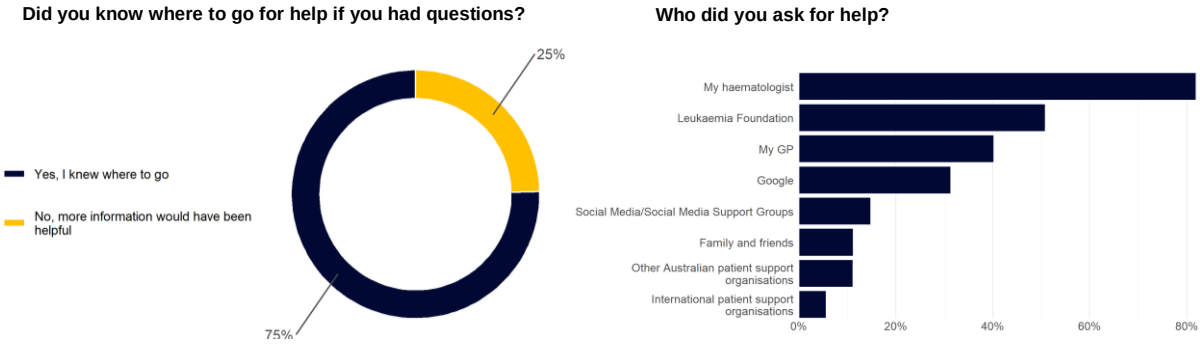
Figure 2.10: In hindsight, would more help in any areas have helped you during active treatment?



Source: People Living with Blood Cancer Survey 2022. See Appendix B.

Patients and members of the blood cancer community continued to highlight the previously reported lack of awareness of availability of services, information and tools to manage side effects when needed. One in four patients did not know where to go to ask for help (Figure 2.11).

Figure 2.11: Patient perspectives – where to go for help



Source: People Living with Blood Cancer Survey 2022. See Appendix B.

During stakeholder consultation, patients cited lack of support in a number of domains (Figure 2.12), including having a limited awareness of availability of peer and nurse support, accommodation support, transport support (such as from government and toll road operators such as Linkt).

Figure 2.12: Patient and stakeholder perspectives on awareness of resources

Source: Insight Economics consultation with stakeholders, conducted in late 2022.

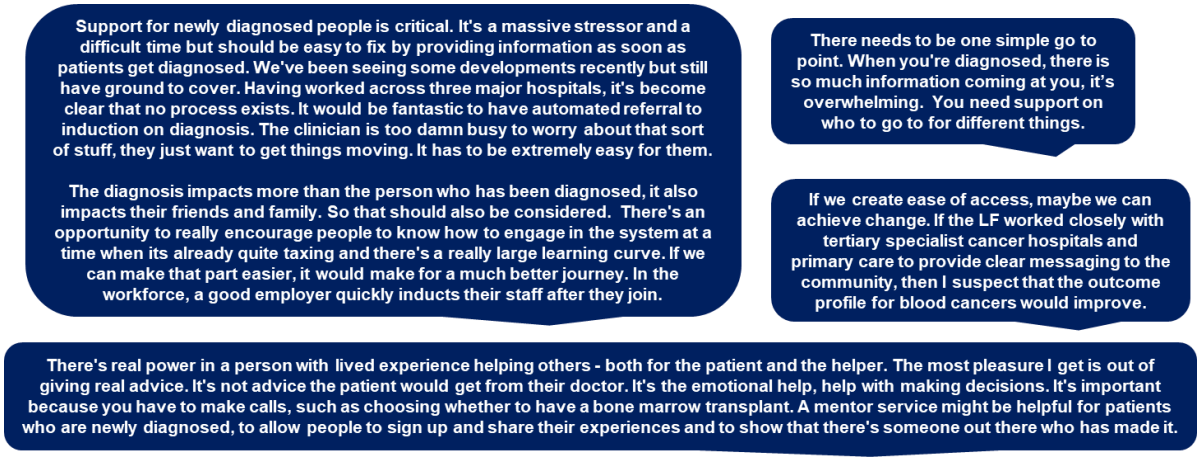
The draft ACP over the next five years aims to implement a holistic healthcare navigation system that is co-designed to underpin personalised care.[29] It will be positioned to address difficulties faced by blood cancer patients in navigating the healthcare system. Cancer Australia has committed to this integrated, co-design model for consumer navigation.

In the design of navigation services, there is a need to focus on the effective engagement of patients at diagnosis. Blood cancer patients have nuanced, disease-specific needs which require specialist support. It will be critical that the consumer navigation and information services are co-designed in a way that caters to these specific needs. Access for triaging and referral to blood cancer support organisations with the expertise and skills to support the specific needs of blood cancer patients are among the highest priorities.

For blood cancers generally and acute blood cancers specifically, there are many critical decisions required from a patient and their family which can have long lasting impacts on their quality of life and potentially the length of their life. Navigating to effective support services at the very beginning of the journey can have life-changing impacts for patients and their carers (Figure 2.13).

[29] Cancer Australia, 2022, Draft Australian Cancer Plan.

Figure 2.13: Stakeholder perspectives on consumer navigation needs



Source: Insight Economics consultation with stakeholders, conducted in late 2022.

Need for informed consent and patient choice in referrals

The initial referral at the primary care level to a specialist can set into motion a series of events that have a substantial impact on long term health and financial outcomes for the patient and their family. All patients need to consider potential out of pocket costs relating to their care and treatment. Patients with private health insurance need to weigh up the benefits of using the private health system versus the potential for out of pocket costs. Supporting patients and their loved ones to make informed decisions at this critical phase is essential.

The first SoTN and NAP identified the need for a complex referral support tool so as to enable patients and GPs to make informed decisions about where they should receive care.[30] As one respondent to the 2022 Survey of People Living with Blood Cancer observed (emphasis added):

> *[We need to] make blood cancer patients like myself and all GPs aware of best clinics to attend that have specialist blood cancer doctors and not just the run of the mill oncologist and haematologists.*
>
> ***Give us a choice** of where to attend not just any random haematologist or oncologist.*
>
> *-Blood cancer survivor*

However, the need for complex referral support has not yet been addressed.

The absence of complex referral support can be explained by several factors. Members of the blood cancer community suggested that data collection is inconsistent across states, meaning that proxies for quality of service are not readily available. For example, in Victoria, there is no data on the volumes of patients seen for a particular subtype by clinician or service, noting that clinicians often work across several hospitals and practices.

Furthermore, members of the blood cancer community indicated that confusion regarding the purpose of referral support prevents adoption. The purpose of complex referral support tools needs to be clarified – these tools aim to address information asymmetry faced by patients and GPs with regard to the most appropriate treatment centre. Complex referral support would provide the data necessary for patients to give informed consent to receive local treatment or seek referral to a specialist centre.

[30] Leukaemia Foundation, 2019, State of the Nation: Blood Cancer in Australia; Leukaemia Foundation, 2020, National Strategic Action Plan for Blood Cancer.

An opportunity exists to empower patients and their GPs to select the best haematologist for them through an informed consent and patient choice registry. The registry could provide patients with information on specialist or treatment center options with simple data with respect to patient volumes by blood cancer subtype, participation in clinical trials and out of pocket costs.

This would complement the development of a National Comprehensive Cancer Centre Network through the ACP.

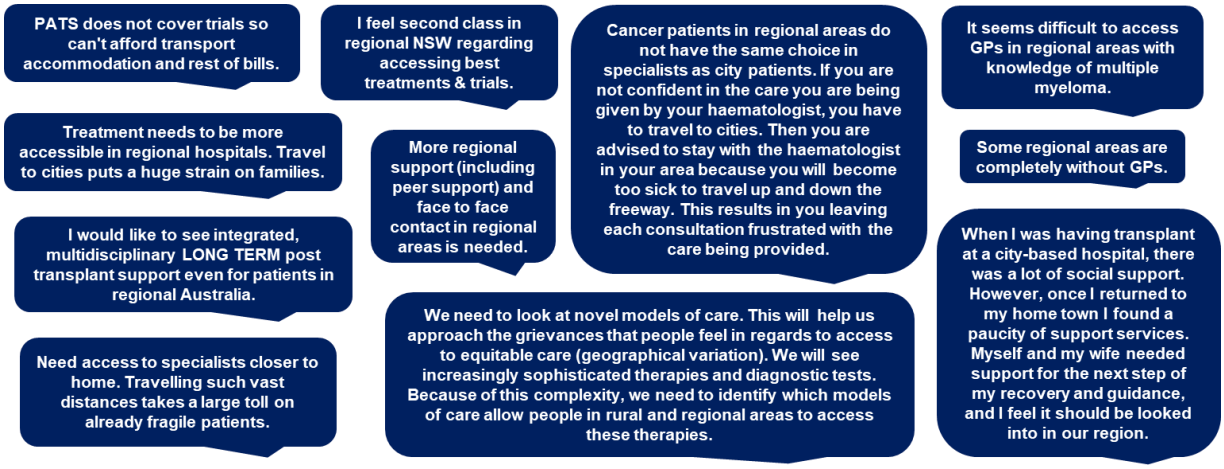
Support for regional and remote patients

Consistent access to best practice treatment and care has the potential to deliver improved survival outcomes and quality of life for people living with blood cancer. State cancer registry data show that survival outcomes at 1-year and 5-years for people living in regional and remote areas are poorer than for metropolitan-based patients. If the metro-regional divide[31] were to be removed, approximately five per cent of expected mortality to 2035 (more than 7,000 deaths over the 2022-2035 horizon) could potentially be avoided.

The causes of disparity are numerous; members of the blood cancer community and patients noted various factors contributing to reduced access (Figure 2.14), including a lack of specialists, specialist diagnostic and blood cancer subtype services, barriers to travel, lack of supportive care and barriers to clinical trial participation. Relatedly, members of the blood cancer community and patients identified the need for improved support through models of care (such as self-administration of medicines) and assisted travel schemes.

While there may be a temptation to adopt highly virtualised support for regional and remote patients, consideration of preferences for face-to-face contact is warranted. Challenges for regional and remote patients is covered in more detail in Chapter 3.

Figure 2.14: Stakeholder perspectives on barriers to access for regional and remote patients



Source: Insight Economics consultation with stakeholders, conducted in late 2022.

2.3 Patient perspectives on priorities for actions to empower patients

The Survey of People Living with Blood Cancer (2022) asked people to rank the four strategic objectives for realising the zero by 2035 vision. The results revealed that improving access to the best quality care was the highest strategic objective for patients, followed

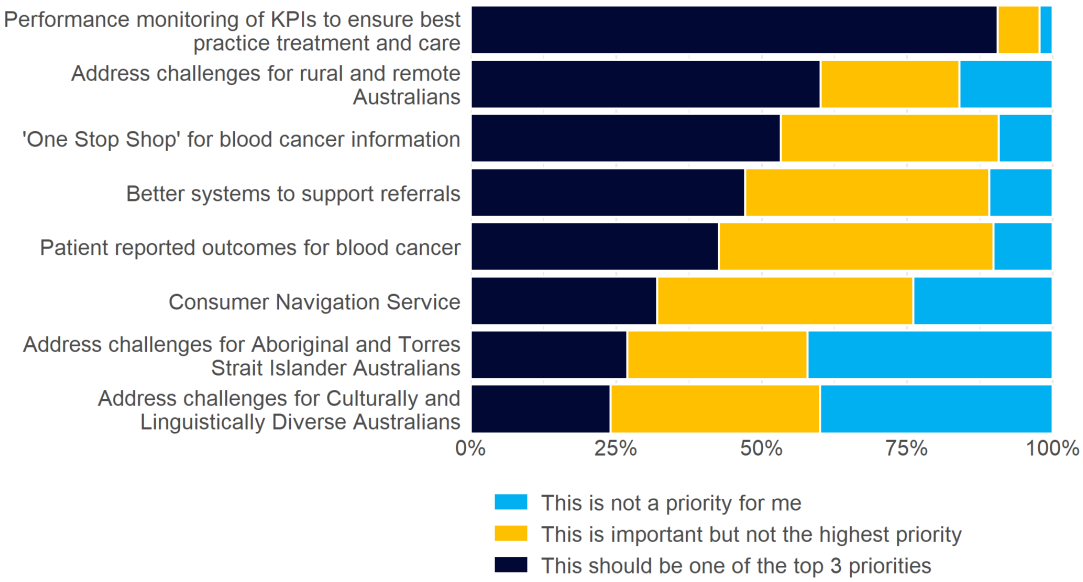
[31] Note in this context, 'regional' includes patients living in regional and remote areas.

closely by funding blood cancer research. Empowering patients and enabling access to novel treatments were ranked as lower-order strategic objectives overall.

This suggests that patients and their loved ones want the health system to work for them – they want to be able to trust and know that the health system delivers the best outcomes without getting lost in the system and act as their own advocates. They also know that research breakthroughs are needed to prevent blood cancers from occurring in the first place, and to realise true cures so that people live longer lives and enjoy a higher quality of life.

Patients who responded to the Survey of People Living with Blood Cancer (2022) were also asked to identify the highest priority actions related to empowering patients, based on the nine actions identified in the NAP (Figure 2.15). Far and away, patients wanted to see a performance framework implemented that ensured patients received quality treatment and care. Performance monitoring can so often be viewed through a negative lens – to identify where the system is going wrong; but it can also be viewed through a positive lens – to build trust in the health system, to show where things are going right. Performance monitoring should be used as a tool to improve the quality and consistency of adherence to OCPs and to build community confidence in Australia's health system.

Figure 2.15: Ranking of strategic objectives by patients



Note:
Ranked from top (highest priority on average) to bottom (lowest priority on average). Source: People Living with Blood Cancer Survey 2022. See Appendix B.

Patients and carers also wanted to see improvements in the health system to address challenges for rural and remote Australians, to improve the availability of information about blood cancer, and better systems to support informed specialist referrals.

2.4 Next horizon priorities

In light of the expected scope of the ACP, the Leukaemia Foundation, in partnership with the Blood Cancer Taskforce, has the opportunity to work with Cancer Australia, federal and state governments and other NGOs to ensure that the major actions of the ACP are implemented in such a way that they meet the needs of blood cancer patients.

A number of key actions identified by the first SoTN and NAP have been taken up by the draft ACP. It is proposed that the Leukaemia Foundation take a partnership role to ensure

implementation of the ACP meets the needs of blood cancer patients against the following key areas of action:

- *Consumer navigation and cancer information service* – Actively engage in the co-design of the service and ensure patients are appropriately triaged to blood cancer support services from diagnosis to get the right support throughout their journey
- *National cancer data ecosystem and performance framework* – Actively engage with Cancer Australia in the design of cancer data frameworks, which would integrate clinical, population and Patient Reported Outcome data, and ensure performance metrics of quality and safety meet the needs of people living with blood cancer (e.g., written care plans, referrals to supportive care).

Areas of need that have been identified by the first SoTN and NAP that are not going to be addressed through other reform efforts include where the Leukaemia Foundation must take a leadership role:

- *Education and training of blood cancer signs and symptoms for GPs* – Work with Cancer Australia to develop disease-specific content that supports improved understanding of blood cancers and blood cancer OCPs
- *Systems for informed consent and patient choice in referrals* – Work with the blood cancer community to develop an informed consent and patient choice directory of blood cancer services
- *Consumer awareness of blood cancer symptoms* – Implement a campaign to improve community awareness of blood cancers and their rights as a patient.

A summary of actions progressed to date and implementation considerations is presented in Table 2.1 and

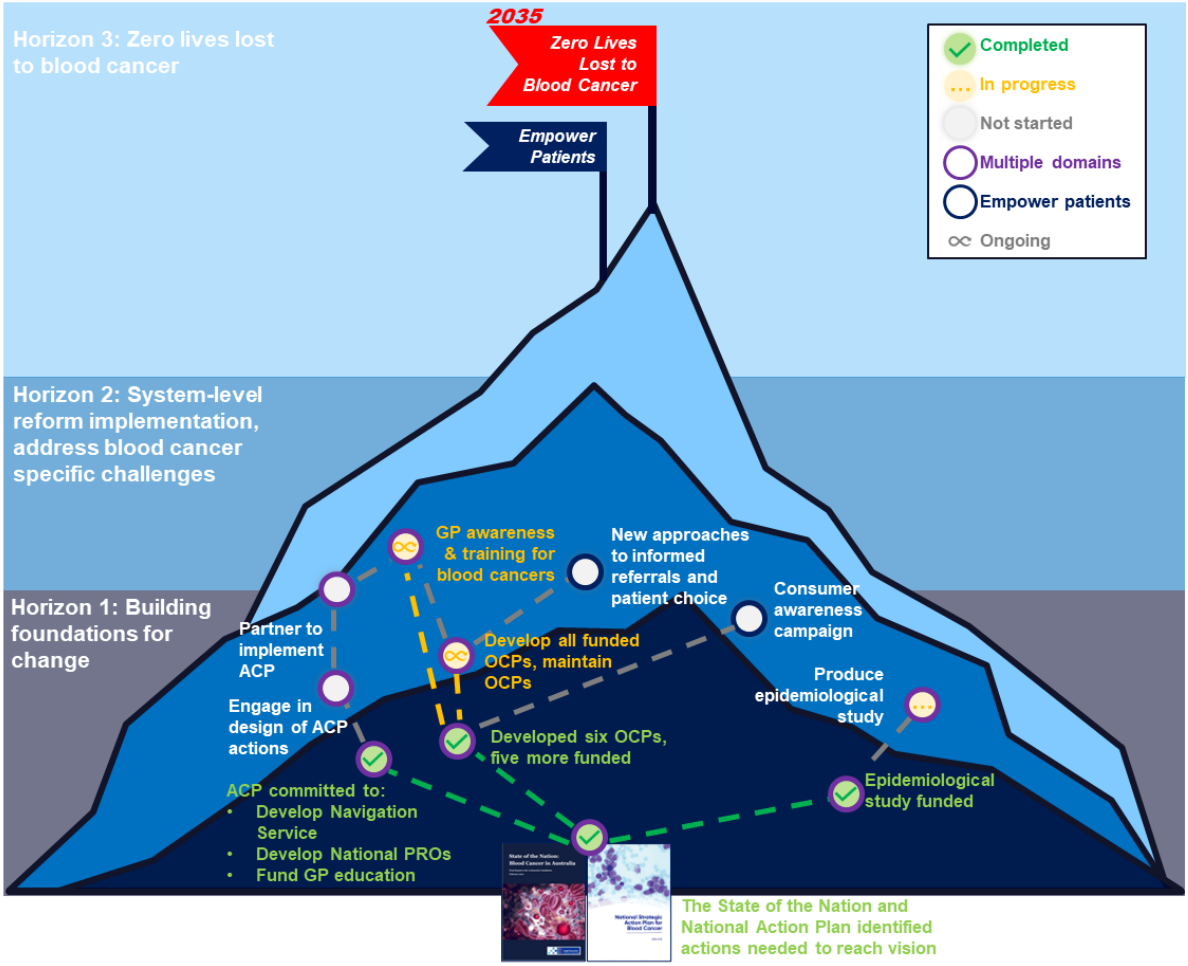
Figure 2.16.

Table 2.1: Summary of actions, progress to date and implementation considerations

Summary of actions	Progress to date	Implementation considerations
Implement a Consumer Navigation Service	✓ National Action Plan endorsement of consumer navigation referral (Action 1.1) ✓ Commitment within Australian Cancer Plan to development of integrated consumer navigation service ✓ Six OCPs developed, five additional funded, which will be the foundation for consumer navigation structure	Reflecting benefits to all cancers and significant funding requirements, but the need to ensure the needs of blood cancer patients are met, it is recommended the Leukaemia Foundation work closely with Cancer Australia in the design and piloting of the integrated consumer navigation service: • Lead: Cancer Australia • Partners: Leukaemia Foundation and blood cancer community
Develop a blood cancer digital health and information strategy – 'create a one stop shop' for trusted blood cancer information	✓ National Action Plan endorsement of consumer navigation referral (Action 1.2) ✓ Commitment within Australian Cancer Plan to development of integrated consumer navigation service and information strategy	
Develop a complex referral support tool for informed referrals and patient choice	✓ National Action Plan endorsement of consumer navigation referral (Action 1.4)	Government stakeholders have communicated constraints in implementing systems that increase information to consumers about provider choice; the Leukaemia Foundation and wider NGO sector have a role to champion improved information for consumers • Lead: Leukaemia Foundation

Summary of actions	Progress to date	Implementation considerations
		• Partners: Cancer Council and blood cancer community
GP education and decision support tools	✓ Commitment within Australian Cancer Plan to increase GP education of cancer through OCP education program	Work with Cancer Australia to develop disease specific content as part of broader education plan. • Lead: Cancer Australia • Partners: Leukaemia Foundation and blood cancer community
Develop a national system for patient reported outcomes (Action 1.5)	✓ National Action Plan endorsement (Action 1.5) ✓ Commitment within Australian Cancer Plan to develop PROs as part of national cancer data ecosystem	Reflecting benefits to all cancers and very significant funding requirements to develop validated, accepted PROs, as well as a commitment to lead this through the Australian Cancer Plan, government is best placed to lead this work, supported by the blood cancer community. • Lead: Cancer Australia • Partners: Leukaemia Foundation and blood cancer community
Address challenges for priority populations	✓ National Action Plan endorsement (Action 1.6-1.8) ✓ Commitment within Australian Cancer Plan to priority populations ✓ Commitment by Leukaemia Foundation to fund epidemiological study	Reflecting benefits to all cancers and significant funding requirements: • Lead: Cancer Australia • Partners: Leukaemia Foundation and blood cancer community

Figure 2.16: Priorities in empowering patients and progress to date



Note: Provides a summary of progress to date and priorities going forward. The following acronyms are used: Australian Cancer Plan (ACP) and Optimal Care Pathways (OCPs). Source: Insight Economics.

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Chapter 3
Catalyse Health Services Reform

Ensuring consistent access to best practice treatment and care has the potential to deliver substantial improvements in survival outcomes and quality of life.

The State of the Nation and National Action Plan observed that we could reduce mortality from blood cancers by removing variations in survival outcomes nationally and between metropolitan and regional (including remote) areas. That is, very substantial improvements could be made – for up to one third of all preventable blood cancer deaths – by implementing known best practice more consistently across Australia. Achieving best practice requires systemic, multilateral, multi-year reform efforts across health settings. To realise these improvements, the State of the Nation and National Action Plan set out key actions to improve the diagnosis, treatment and care of people living with blood cancer.

This chapter briefly reviews the current landscape for promoting implementation and adoption of known best practice, looks at actions undertaken and considers priorities for the next horizon.

3.1 Achievements to date

Ensuring consistent access to best practice treatment and care has the potential to deliver substantial improvements in survival outcomes and quality of life.

Based on the most recent state cancer registry data for survival outcomes by age, sex, region and blood cancer sub-type, it is estimated that blood cancer mortality could be reduced by 29 per cent if best practice outcomes were achieved nationally. Over the 2022-2035 horizon, this would equate to 38,200 fewer deaths.

The NAP observed challenges to achieving best practice, including accuracy of diagnosis, variation in clinical practices, inconsistent access to supportive care, workforce challenges and financial hardship for patients and their loved ones.

Since the launch of the NAP, and as explained in detail in Section 2.1, major achievements to date which act to address challenges in achieving best practice include:

- Development of **six OCPs in partnership by the Blood Cancer Taskforce**, and government commitment to funding five more OCPs to be delivered in mid-2023
- Development of a **clinical guideline for blood cancer**
- Development of a **national cancer data ecosystem and performance framework**
- Increased **engagement with GPs in the signs and symptoms of blood cancer** and the use of the OCPs
- Secured funding for **future GP education into the signs and symptoms of cancer as part of the implementation of the ACP**
- Federal Government to develop **national system for Patient Reported Outcomes** in cancer.

In addition, the draft ACP includes recommendations of important actions to develop a **National Comprehensive Cancer Centre Network**, which is aimed at improving the quality and safety of care across Australia, underpinned by a performance monitoring framework (Box 3.1) and innovative, evidence-based **models of cancer care**.

Box 3.1: The development of a National Comprehensive Cancer Centre Network

An integrated, coordinated system of cancer care is critical to delivering optimal cancer care and improving outcomes across the cancer care continuum. A national network of comprehensive cancer centres has the potential to strengthen collaboration across jurisdictions, centres of excellence and other cancer and health services. Key functions may include knowledge sharing, standardising person-centred clinical care and practices, enabling research-driven excellence across the cancer care continuum, strengthening transparency of reporting, governance, and translation of research, and enabling evidence- based system performance improvement.

The first step will involve the development of a national framework for networked comprehensive cancer care, including:

- To define the role of comprehensive cancer centres in enhancing patient outcomes, strengthening transparency and accountability, and driving continuous improvement.
- To establish a National Comprehensive Cancer Centre Network (NCCCN) with clearly defined roles and accountability mechanisms both within cancer care and the broader health system, across the cancer continuum from prevention and early detection to treatment, survivorship and end-of-life.

The establishment of a NCCCN could:

- Promote collaboration between Australia's leading cancer centres, researchers, and specialists to deliver world-class, equitable, accessible, and evidence-based cancer care for all Australians, regardless of location, background or cancer type.
- Strengthen links between comprehensive cancer centres and drive collaboration between and within jurisdictions, as well as partnering with the primary care sector and health districts.
- Extend ongoing monitoring of system performance across the network and individual centres within the network to identify centres of excellence and areas warranting improvement, including ongoing development of workforce and research priorities. It is important that the NCCCN is based on standards for research as well as clinical outcomes with an emphasis on person-centred care.
- Establish and maintain a clear governance model for the effective management and leadership of centres, with enough flexibility to accommodate different structures and settings across the NCCCN. This action may also incorporate the development of a framework and guidelines on how cancer centres can join the NCCCN, minimum standards and requirements for monitoring and improvements.
- Monitoring system performance will ensure that evidence-based, transformative research and care conducted through the NCCCN is expanded to all Australians, improving equity of access across services for all consumers.

The establishment of any performance standards or guidelines should be produced in collaboration with priority population groups, particularly Aboriginal and Torres Strait Islander people, to ensure that the needs and cultural safety of all people is prioritised in the delivery of services through the NCCCN. This action could also ensure distribution of the NCCCN across Australia, including rural, remote, and low socioeconomic areas to ensure equity.

Source: Cancer Australia, 2022, Draft Australian Cancer Plan.

National workforce strategies are also intended to be implemented through the ACP, noting that workforce challenges have been exacerbated by the COVID-19 pandemic, which has resulted in significant burnout and turnover. The draft ACP includes 2-year actions to:

- Identify current and emerging workforce undersupply in line with cancer workforce modelling and other national health workforce strategies, and initiate planning with the sector towards building future workforce capacity and capability

- Build on existing capability of the primary care workforce to collaboratively and sustainably meet the needs of consumers
- Evaluate and extend cultural safety training programs to cancer service providers, including community-based partnerships with priority population groups.

Within 5 years, the draft ACP aims to:

- Implement a cancer care workforce pipeline that meets demand for optimal cancer care, with diversity measures in training, recruitment and talent management to ensure the cancer workforce represents the diversity of patient populations
- Work with the sector to support all cancer care practitioners to practice at the top of their scope of work, increase retention and ensure ongoing access to continuing professional development
- Integrate routine cultural safety training programs to cancer service providers, including community-based partnerships with priority population groups.

3.2 Key areas of need

Achieving clinical best practice (i.e., via catalysing health service reform) is the highest strategic objective for patients, of which no single action alone can deliver. Rather, a series of reforms that work together are needed, almost all of which require multilateral collaboration and funding to execute. Key areas of need include:

- Improving timely blood cancer diagnosis and informed referrals
- Improving the accuracy of diagnosis by making genomics
- Defining models of care and clinical standards for blood cancer treatment and care
- Investing in workforce capacity and skills development
- Implementing reforms to prevent financial toxicity.

Improving timely diagnosis and informed referrals

Stakeholder consultation and responses to the 2022 Survey of People Living with Blood Cancer indicate that poor GP awareness of blood cancer signs and symptoms contribute to delays in diagnosis and referral (Chapter 2, Figure 3.1).

General Practitioners play a central role in ensuring patients access high quality care. In blood cancer care specifically, the role of GPs could increase due to new treatment modalities and long-term management needs. Likewise, tertiary centres are piloting shared care models to explore localised care.[32] However, as highlighted by the Royal Australian College of General Practitioners, general practice is in crisis:[33]

- Almost three in four GPs reported feelings of burnout over the past 12 months
- Almost one in two GPs reported that it is financially unsustainable for them to continue working as a GP, and 70 per cent of practice owners indicated concern about the ongoing viability of their practice
- While one-quarter of GPs reported that they plan to retire within the next five years, only 14 per cent of medical students indicated general practice as their preferred medical specialty.

[32] See, for example: McLoone, J.K., Weihan, C., Wakefield, C.E., et al., 2022, Childhood cancer survivorship care: A qualitative study of healthcare providers' professional preferences, Front. Oncol. 12, 945911, doi:10.3389/fonc.2022.945911.
[33] Royal Australian College of General Practitioners, 2022, General Practice Health of the Nation.

Increased demands to provide patient care, diagnosis and treatment due to the pandemic has resulted in a widely frustrated and stretched area of general practice who are calling for urgent and investment and support. The role of GPs in blood cancer diagnosis and treatment is undergoing change with the introduction of more frequently used oral therapies and the increase in long-term management of cancer conditions. In response to these trends, tertiary centres are experimenting with pilots of shared care models which enable patients to access more localised care. However, to achieve such transformations in models of care in the diagnosis and treatment of blood cancers requires an increased need for the education and support of GPs, to achieve an improved knowledge of blood cancers when patients present. In consultations, the blood cancer community endorsed calls for investment in the workforce and new approaches to supporting GPs.

Considering the broader challenges facing primary care, other complimentary actions could be considered to improve the timely diagnosis and referral of blood cancer patients. These could include investment in improved decision support tools for GPs and the development of the blood cancer workforce. While this does not address GP retention, it may assist in more timely diagnosis by making decision pathways easier for GPs to decipher. The Australian Digital Health Agency is notionally supporting the development of digital support tools (Box 3.2),[34] but more work could be put towards better supporting this already stretched but critical pillar of Australia's health system.

Box 3.2: Supporting GPs through enhanced digital platforms

Computerised clinical decision support systems can support more health care being provided by generalists, augmenting knowledge in complex decision making. Decision support systems have been in use since the 1980s, but are now developing rapidly, and have been recommended for use in Australia for some areas of practice, such as diagnostic medicine, by the Medicare Benefits Schedule Review Taskforce.

This is an important area of evolving digital capability that can harness software systems to support doctors to more easily keep up with developing areas of clinical practice (such as treatment pathways for certain types of cancers, or new medicines available to treat diabetes).

Clinical software has already been used to reduce workload for doctors and improve safety outcomes for patients and communities through support for active ingredient prescribing. This came into effect under PBS legislation from 1 February 2021.

Upgrading and developing clinical software to promote interoperability between primary care, hospital and aged care clinical information systems is a continuing area of focus for the Australian Digital Health Agency and all governments.

Under this Strategy, stakeholders will collaborate to consider where incorporating decision support software into clinical systems may assist generalists to practise safely with expanded scope, and to ensure the development of software and algorithms that accurately reflect safe, evidence-based clinical guidelines and treatment pathways, and continuing referral of patients to more specialised care when appropriate.

Source: Department of Health, 2021, National Medical Workforce Strategy 2021–2031, accessed at: https://www.health.gov.au/sites/default/files/documents/2022/03/national-medical-workforce-strategy-2021-2031.pdf, p 65.

The draft ACP includes considerations for GP education developed around the OCPs. This is a welcome first step. There will be the opportunity for the Leukaemia Foundation to partner with Cancer Australia, and federal and state governments to ensure this education program improves awareness and understanding of the signs and symptoms of blood cancer.

Critically, improving and integrating information to support informed referrals and patient choice is a high priority for blood cancer patients and carers.

[34] Department of Health, 2021, National Medical Workforce Strategy 2021–2031, accessed at: https://www.health.gov.au/sites/default/files/documents/2022/03/national-medical-workforce-strategy-2021-2031.pdf.

Figure 3.1: Perspectives on delayed diagnosis (highlighted in survey and consultations)

Source: Insight Economics consultation with stakeholders, conducted in late 2022.

Improving the accuracy of blood cancer diagnoses

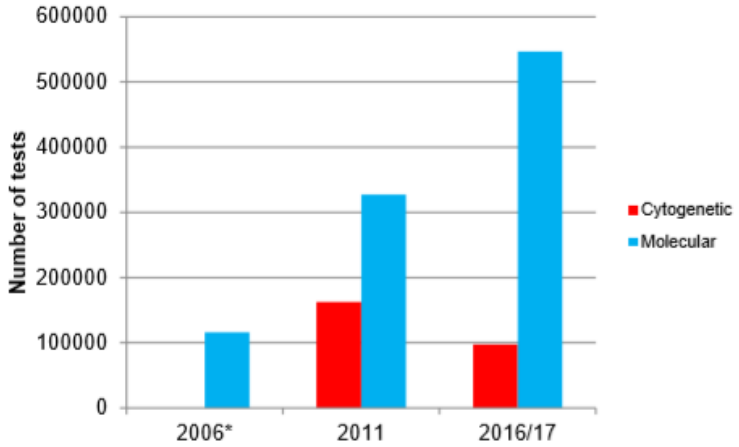
Access to accurate and timely diagnostics is critical to the treatment and care of a person living with blood cancer. As the complexity of blood cancers is better understood, so too are the diagnostic tools used to diagnose and treat a patient.

Generally, a patient with blood cancer will present within a primary care setting, most often a GP, with ambiguous symptoms, such as fatigue, bruising or pain, that may be associated with a range of conditions. The GP will order a full blood examination which will be delivered by a pathologist. If the test results indicate a blood cancer, the GP will refer the patient to a specialist to have a series of tests to confirm diagnosis; the specific diagnostics required vary by blood cancer sub-type, tests ordered by a specialist to confirm diagnosis have traditionally included:

- Physical examination and blood tests
- Imaging including: x-ray, computed tomography (CT) scan – a specialised x-ray for building up three-dimensional pictures of the body, and positron emission tomography (PET) scan – a small amount of radioactive material is injected, which highlights cancerous areas when viewed with a special scanner
- Bone marrow aspiration, which involves the removal of liquid bone marrow using a syringe, or bone marrow trephine biopsy which involves the removal of a 1 or 2cm core of bone marrow in one piece
- A lymph node biopsy.

However, cytogenetic and molecular genomic testing are increasingly used alongside these tests to provide a more accurate diagnosis by sub-type. This additional genomic information about an individual's cancer can help inform prognosis and treatment planning. Reflecting the value of these tests, the volume of cytogenetic and molecular testing has increased exponentially since 2006 (Figure 3.2).

Figure 3.2: Cytogenetic and molecular test volumes (2006, 2011, 2016-17)



Source: RCPA, 2018, *Health Genomics Survey 2017*, Final Report, p 28 (latest report).

In addition, a range of other novel diagnostics are in development that offer the potential to inform patient prognosis and treatment planning, as well as the selection of targeted therapies. Major examples include:

- Next generation sequencing, which represents a high throughput approach to DNA sequencing and is an effective way to capture a large amount of genomic information about a cancer

- Minimal residual disease testing, which involves looking for very low levels of blood cancer using highly sensitive methods of testing.

These diagnostic tools have become standards of care in comprehensive cancer centres globally; the World Health Organisation's (WHO) classification of tumours of haematopoietic and lymphoid tissues, which is used to guide diagnosis, prognosis and the selection of appropriate therapies, is predicated on integrating morphologic (cytology and histology), immunophenotypic, molecular and cytogenetic data.[35]

In alignment with global consensus, the NAP called for precision medicine (with genomic testing as required) to become the standard of care. Members of the blood cancer community reaffirmed the need for genomics to become the standard of care (Figure 3.3).

Figure 3.3: Perspectives regarding precision medicine becoming the standard of care

Source: Insight Economics consultation with stakeholders, conducted in late 2022.

Despite its importance, analysis of responses to the 2022 Survey of People Living with Blood Cancer highlights that few patients currently undergo genomic testing; only one in five patients (21%) knew that a genomic test was used to confirm their diagnosis (43% did not know if a genomic test was used to confirm their diagnosis). Of those who underwent a genomic test, **31 per cent said that the genomic test changed their diagnosis and treatment plan**—the first time that this survey data has been captured.

As explored in the NAP, major challenges also exist in the delivery of diagnostic services for blood cancers that are defined as minimum standards of care by blood cancer clinical guidelines.[36] Revision rates in genomic testing can be high, particularly at non-academic

[35] Khoury, J.D., Solary, E., Abla, O., et al., 2022, The 5th edition of the World Health Organization Classification of Haematolymphoid Tumours: Myeloid and Histiocytic/Dendritic Neoplasms, Leukemia, 36, 1703–1719, doi: 10.1038/s41375-022-01613-1; Alaggio, R., Amador, C., Anagnostopoulos, I., et al., 2022, The 5th edition of the World Health Organization Classification of Haematolymphoid Tumours: Lymphoid Neoplasms, Leukemia 36, 1720–1748, doi: 10.1038/s41375-022-01620-2.

[36] Leukaemia Foundation, 2021, National Strategic Action Plan for Blood Cancer.

treatment centres,[37] and timeliness remains a significant concern for patients and clinicians. For example, the median reporting time for fluorescence in situ hybridisation (FISH) testing has been reported to be nine calendar days, but the range for reporting time for cancer patients was reported to be anywhere from one to 56 calendar days.[38] Members of the blood cancer community indicated that this may be a conservative estimate, with FISH testing for myeloma reported to routinely require 'up to six months'.

Addressing funding gaps and service delivery challenges in diagnostics is a high priority in realising best practice and the zero by 2035 vision. In addition, the Zimmerman Report called for the Federal Government to fund, in partnership with states and territories, a nationwide genomics testing program run through a Centre for Precision Medicines and Rare Diseases (CPMRD).

This recommendation is applauded and, as explored in Chapter 5, could be implemented as part of a wider evidence harmonisation strategy.

It is noted, however, that adoption of current precedent could speed up availability of genomic and genetic testing to blood cancer patients. This would follow from adoption of precedent established by MSAC, in which WHO guidelines were leveraged as an appropriate standard of care for Australian patients; at its August 2019 meeting, in support of approval of genetic tumour testing applications 1526, 1527 and 1528, MSAC noted that:[39]

> *The current WHO Guideline on lymphoid neoplasms and IMWG consensus on risk stratification in multiple myeloma provide the appropriate standards of clinical care for Australian patients:*
>
> *The Department and applicant had agreed an approach to the determination of clinical utilities for each of the proposed tests, based on a triage assessment developed prior to, and discussed at, the Pathology Pilot Meeting… The entry of each test in the WHO Guideline was accepted to provide sufficient demonstration of its diagnostic performance, and also its clinical validity and/or clinical utility.*

Defining models of care and clinical best practice for blood cancer

Substantial progress has been made in the development of OCPs for blood cancers, but more work is needed to develop Australian clinical guidelines for best practice care. By bringing together available evidence to underpin scientifically valid recommendations for the diagnosis, treatment and care of patients, respected and well adopted clinical practice guidelines work to improve patient outcomes.[40]

While the benefits of clinical guidelines are significant, Australia does not have national guidelines for blood cancer in contrast to other developed nations, including National Comprehensive Cancer Network (NCCN) guidelines in the United States, National Institute of Care Excellence (NICE) guidelines in the UK and European Society for Medical Oncology (ESMO) guidelines which inform clinical practice in Europe. Some guidelines have been philanthropically developed and are in use at specialist centres, such as the guidelines for

[37] While data on Australian revision rates are not available, National Action Plan working group members indicated challenges in Australian clinical practice mirrored overseas evidence; see for example: Bowen, J.M., Perry, A.M., Laurini, J.A., et al,, 2014, Lymphoma diagnosis at an academic centre: rate of revision and impact on patient care, British Journal of Haematology, 166, 202-208, doi: 10.1111/bjh.12880.

[38] FISH refers to a cytogenetic technique which enables targeting of specific chromosomal locations within the nucleus of a cell. RCPA, 2018, *Health Genomics Survey 2017*, Final Report, p 55.

[39] Medical Services Advisory Committee, 2019, Application No. 1526 – Somatic gene testing of haematological malignancies.

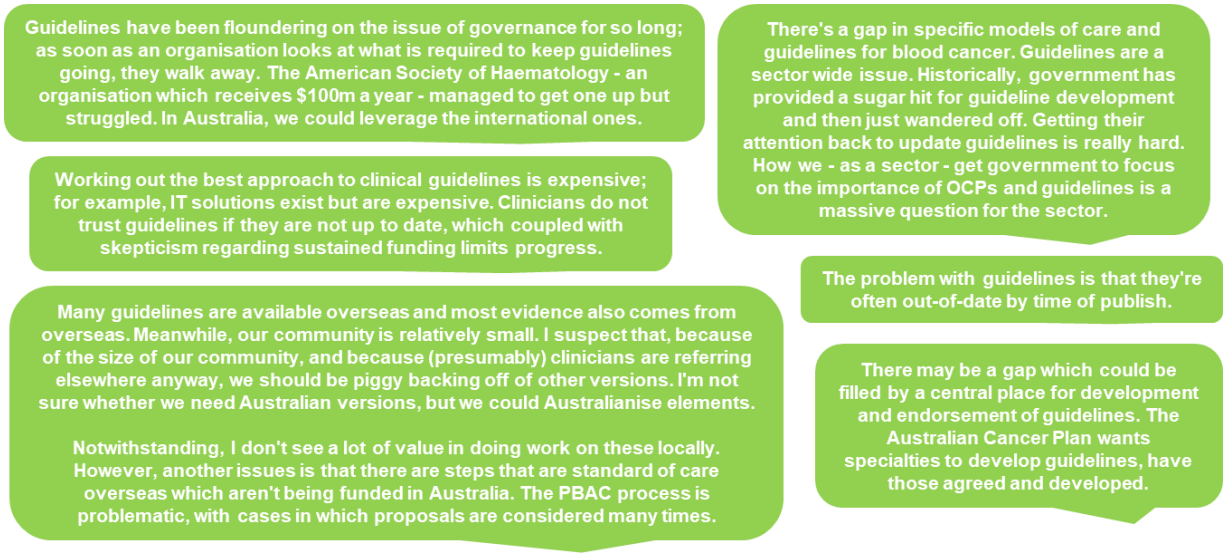
[40] Cancer Council Australia, 2020, Clinical Practice Guidelines, accessed at: https://www.cancer.org.au/health-professionals/clinical-guidelines/; Harrison, R., Manias, E., Mears, S., et al., 2019, Addressing unwarranted clinical variation: A rapid review of current evidence, J Eval Clin Pract, 25; Australian Commission on Safety and Quality in Health Care, 2018, Clinical Care Standards; Wong Doo, N., White, V.M., Martin, K., et al., 2019, The Use of Optimal Treatment for DLBCL is Improving in All Age Groups and Is a Key Factor in Overall Survival, but Non-Clinical Factors Influence Treatment, Cancers, 11(928), doi: 10.3390/cancers11070928.

myeloma treatment and care developed by Myeloma Australia, but gaps exist for other blood cancer sub-types.

The cost of developing and maintaining clinical guidelines generally exceeds the NGO sector's funding capabilities, and yet, responsibility for the development of clinical guidelines has fallen to this sector. This results in an *ad hoc* and inefficient approach to guidelines development without a clear process for national endorsement nor any defined framework on how or when they should be updated. Many groups develop consensus-based guidelines, which are faster and cheaper to develop than evidence based guidelines but are often either not evidence based or supported by limited evidence.[41]

Some government stakeholders questioned whether Australian guidelines are needed and suggested that perhaps Australia could continue to 'free-ride' on international guideline efforts (such as World Health Organisation guidance; Figure 3.4). This becomes problematic when international guidelines are not updated or recommend therapies not available in Australia. Moreover, the absence of clinical guidelines makes applications for regulatory approval and reimbursement all the more challenging.

Figure 3.4: Stakeholder perspectives on the need for clinical guidelines



Source: Insight Economics consultation with stakeholders, conducted in late 2022.

The complexities associated with clinical guideline development necessitate a systemic approach which:

- Benefits from economies of scale in process (single body responsible for developing clinical guidelines in cancer, such as eviQ)
- Benefits from specialist and community input (e.g., drawing upon nominees from the Blood Cancer Taskforce), to promote support and adoption by clinicians
- Is updated frequently (possibly living) to reduce risk of dismissal.

The Federal Government has funded the development of one clinical guideline for blood cancer through the Phase 1 implementation of the NAP, which is currently underway. This is an important first step, and opens up a conversation about how best to achieve a sustainable solution to the development, governance and maintenance of guidelines.

[41] Venus, C., Jamrozik, E., 2020, Evidence-poor medicine: just how evidence-based are Australian clinical practice guidelines? Intern Med J, 50(1), 30-37, doi: 10.1111/imj.14466.

Australia should develop a national approach to clinical guidelines in cancer, approaching clinical guidelines as core enabling infrastructure for reducing variation in clinical care, improving health outcomes and improving the cost-effectiveness of services. A centralised, national approach should be adopted and endorsed to obtain process efficiencies and promote consumer trust and adoption.

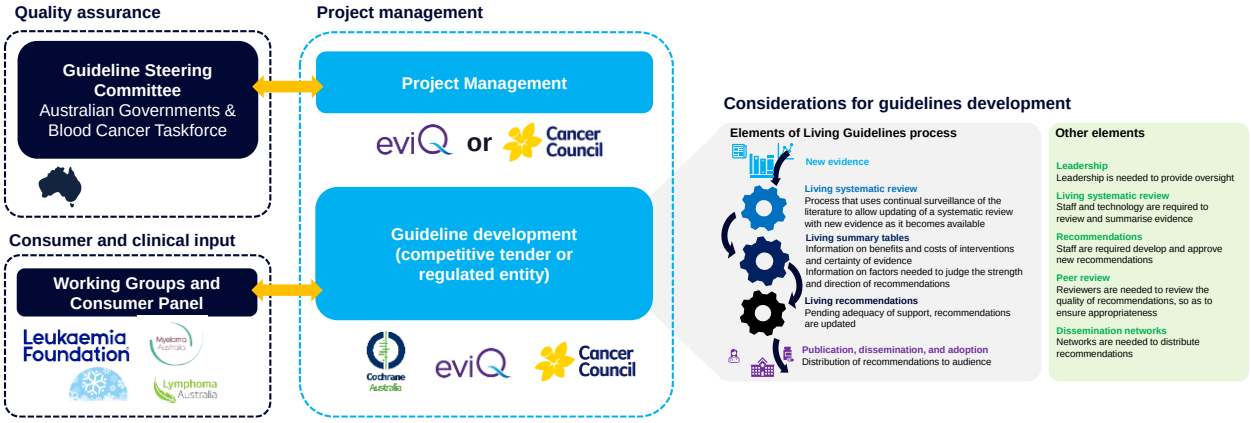
Cancer Australia was established by the Federal Government in 2006 to lead and coordinate a national, evidence-based intervention across the continuum of care. However, since implementation, government has demonstrated its preferred model is to act in a funding capacity and outsource this work to other organisations. This has been demonstrated through the approach to OCPs, to the blood cancer clinical guideline and more broadly in the development of living guidelines for other conditions such as stroke management, diabetes, pharmacological management of inflammatory arthritis, and COVID-19.[42]

Within the domain of cancer, funding for guideline development could be provided to an independent national organisation. This could be either via:

- A government agency, such as eviQ, which is hosting guidance for evidence-based, consensus driven cancer treatment protocols and information, or
- The Cancer Council, which is hosting the OCPs.

The organisation developing the guideline would liaise with the Blood Cancer Taskforce for clinical expertise and with NGOs, such as the Leukaemia Foundation, for patient input to clinical guideline development. Figure 3.5 provides an overview of a potential model for a national approach to the development and governance of clinical guidelines.[43]

Figure 3.5: A national approach to the development and governance of clinical guidelines



The development of a national cancer data ecosystem, properly conceived and designed, should allow for monitoring of adherence to clinical best practice against these guidelines and identification of practice improvements; this is understood to be the aim of the cancer data ecosystem to be developed by Cancer Australia.

Workforce shortages extend beyond primary care

Australia's propensity to deliver improvements in outcomes is limited by the capacity of Australia's healthcare workforce. An underequipped workforce will not be able to adopt best

[42] Cochrane Australia, 2018, In a world-first, 'living stroke guidelines' are set to be piloted in Australia, Australian Living Evidence Consortium website, accessed at: https://livingevidence.org.au/new-index-3; English, C., Hill, K., Cadilhac, D.A., et al., 2022, Living clinical guidelines for stroke: updates, challenges and opportunities, Med J Aust, doi: 10.5694/mja2.51520.
[43] To achieve value for money, funding models including competitive tender or ex-ante regulation should be explored.

practice or be able to maximally contribute to improvements in best practice, without spillover effects on wellbeing.

Consistent with mega-trends towards short-run efficiency in favour of resilience, patients and clinicians suggested that the healthcare workforce is inadequately resourced. COVID-19 was 'the straw that broke the camel's back', contributing to 'the decimation of the health workforce nationally' (Figure 3.6).

Various reform and policy priorities are aiming to address workforce issues; for example, the draft ACP aims to develop:[44]

> *... an engaged, capable, and future-focused cancer workforce that is culturally safe and responsive, well-equipped, well-supported and driven by collaboration, continuous improvement, and diversity to enable inclusive care for all Australians...*

Notwithstanding, representatives from the blood cancer community raised concerns that blood cancer specific workforce issues will not be addressed by broad reform. Without adequate resourcing, there is real risk of high-cost health care and lost opportunities for improvements in outcomes. For example, clinician stakeholders indicated that today's workforce is inadequately resourced to deliver novel medicines and CAR T-cell therapies, meaning Australia's propensity to adopt this technology in a sustainable manner is presently limited (Figure 3.6).

Figure 3.6: Stakeholder perspectives on the integrity of Australia's health workforce

Source: Insight Economics consultation with stakeholders, conducted in late 2022.

In order to address workforce issues, NGO stakeholders highlighted the need for investment in research infrastructure, workforce, processes/incentives, and models of care:[45]

- National training programs
- Retention strategies
- Career pathways
- Next generation recruitment and training preparation for altered models of care
- Ongoing commitment to upskilling the Australian cancer workforce
- IT resources to enable electronic prescribing.

[44] Cancer Australia, 2022, Draft Australian Cancer Plan.
[45] ALLG & HZANZ, 2022, *The Australian Cancer Plan 2023–2033 consultation*, Submission.

Preventing financial hardship

As outlined in Chapter 1, the financial effects of blood cancers can be dramatic - often combining out of pocket costs and temporary relocation for treatment with inability to work. Challenges relating to financial hardship identified in the first SoTN, include:[46]

- Poor coverage of private health insurance, coupled with limited informed consent, which contributes to unanticipated and potentially exorbitant financial costs
- Complex processes for accessing government support, which contributes to delayed access
- Limited availability and extent of support, which contributes to out of pocket expenses or failure to supplement loss of disposable income
- Limited awareness of eligibility for assistance, which contributes to patients incurring out of pocket costs which could otherwise have been covered.

Review of responses to the patient survey indicates that these issues persist (Figure 3.7).

Figure 3.7: Patient and stakeholder comments on financial costs

Source: Insight Economics consultation with stakeholders, conducted in late 2022.

Access to travel and accommodation support continues to be an issue faced by patients. Eighteen per cent of the 1,321 survey respondents who indicated that they needed transport and accommodation support were unable to access it. Concerns regarding access to transport and accommodation support raised by the blood cancer community include limited support for switching carers and limited support for larger families. As one researcher noted, these issues loom large for the Aboriginal and Torres Strait Islander community:

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

[46] Leukaemia Foundation, 2019, *State of the Nation: Blood Cancer in Australia*.

- Researcher.

Considering the substantial financial impact of a blood cancer diagnosis, the NAP calls for:[47]

- Reform of Patient Assisted Travel Schemes (PATS), including advocating for streamlined administrative processes, greater access to the schemes and support for patients to participate in clinical trials.
- Review of options for the introduction of financial support mechanisms (such as a temporary disability payment) to support people with cancer and other serious illness who require temporary financial support.

The NAP's call to action is consistent with needs expressed by patients (Figure 3.8), including:

- Increased availability of government support, such as healthcare cards, Centrelink or NDIS
- Reduced barriers and more timely access to government support, through easier and swifter application processes and increased availability of financial assistance for patients regardless of employment status of partners.

Figure 3.8: Patient calls for financial support to meet financial needs

Source: Insight Economics consultation with stakeholders, conducted in late 2022.

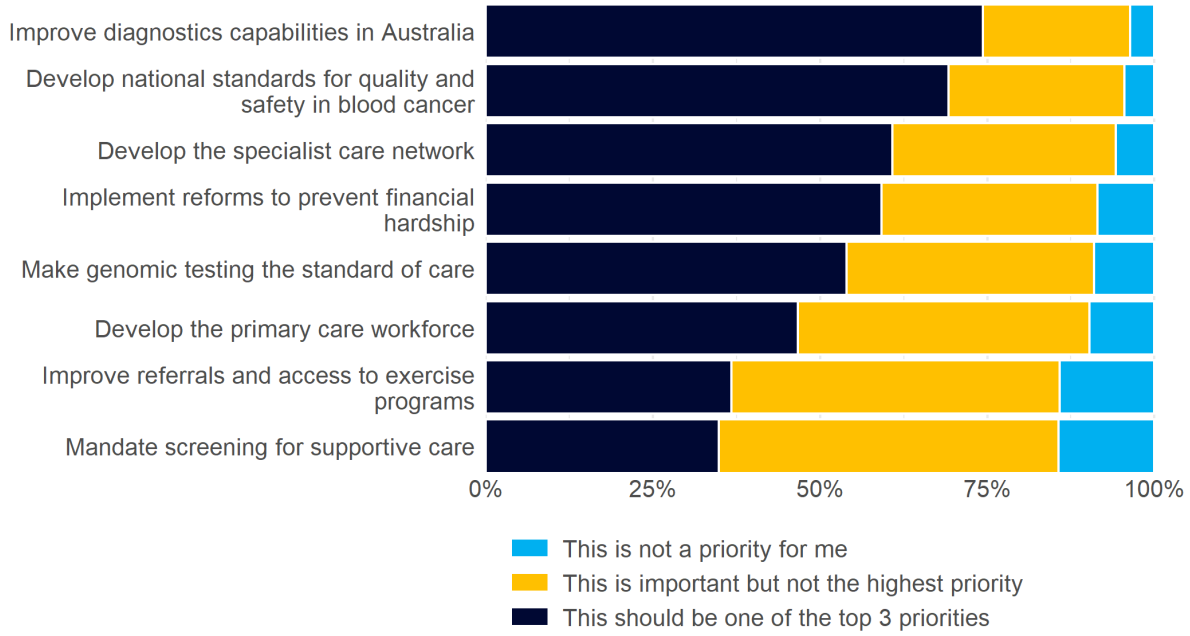
[47] Leukaemia Foundation, 2020, National Strategic Action Plan for Blood Cancer.

3.3 Patient perspectives on priorities for achieving best practice

As noted in Chapter 2, the Survey of People Living with Blood Cancer revealed that improving access to best practice care was the highest strategic objective for patients.

Within the strategic objective to catalyse health services reform and enable access to best practice care, the highest priorities for patients was to improve diagnostics capabilities for blood cancers (Figure 3.9). This demonstrates that consumers understand that best practice care begins with an accurate and timely diagnosis and reflects concerns that there are gaps in Australia's capabilities in this domain.

Figure 3.9: Ranking of strategic objectives



Note: Ranked from top (highest priority on average) to bottom (lowest priority on average). Source: People Living with Blood Cancer Survey 2022. See Appendix B.

The next highest priority was accountability through national standards for quality and safety in blood cancer. Patients want the system to work for them, and they want to be able to trust the system to consistently deliver best practice care. This was followed by the development of a national specialist care network, reforms to prevent financial hardship and making genomic testing the standard of care.

3.4 Next horizon priorities

Many of the priorities for patients and key areas of need are intended to be tackled by implementation of the ACP, which includes:

- The development of a National Comprehensive Cancer Centre Network
- Strategies to address cancer workforce challenges
- GP education to promote awareness and uptake of the OCPs, including signs and symptoms of cancer and referrals
- The development of a national cancer data ecosystem and performance framework with metrics of quality and safety.

The Leukaemia Foundation can take a **partnership role** to ensure that implementation of these ACP initiatives meets the needs of blood cancer patients and help deliver the vision for 2035.

The Leukaemia Foundation should also work in partnership with the Cancer Council and wider NGO community to tackle reforms to reduce financial hardship; this is a lead priority for the Cancer Council.

However, there remain areas of need that have been identified by the first SoTN and NAP which are not going to be addressed through other reform efforts. The Leukaemia Foundation has a leadership role to play in addressing these challenges, including:

- *Support the development of blood cancer models of care and clinical guidelines for blood cancer* – Build on Cancer Australia's work to develop innovative models of care within the National Comprehensive Cancer Network to define models of care and develop additional clinical guidelines for blood cancer in partnership with the Blood Cancer Taskforce
- *Champion a national approach to clinical guidelines governance and funding* – Working with the Blood Cancer Taskforce, and building on the Phase 1 funding for the development of a blood cancer clinical guideline, the Leukaemia Foundation should advocate for an efficient and sustainable, national approach to clinical guidelines development.

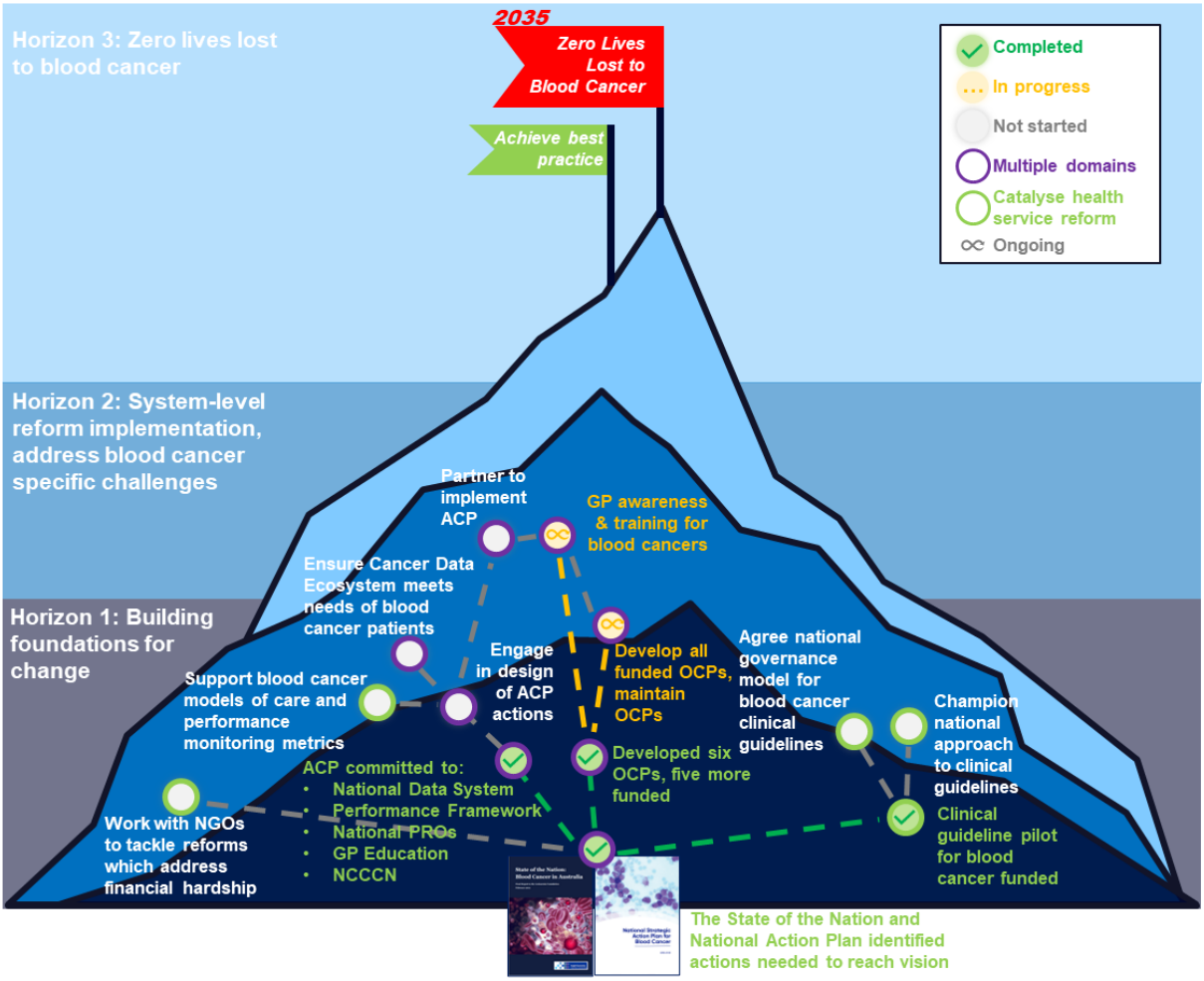
A summary of actions progressed to date and implementation considerations is presented in Table 3.1 and Figure 3.10.

Table 3.1: Summary of actions, progress to date and implementation considerations

Summary of actions	Progress to date	Implementation considerations
Address OCP and clinical guideline gaps	✓ Six OCPs developed, five additional funded ✓ National Action Plan recommendation to develop clinical guidelines ✓ Funding for pilot approach to clinical guideline development	National approach needed to develop sustainable model for clinical guideline funding and governance. Recommended that a national body be funded to develop and maintain clinical guidelines, such as eviQ or Cancer Council Australia. The Blood Cancer Taskforce can serve as a clinical and patient expert reference group to guideline development.
KPIs for quality, safety and patient experience, including use of MDTs, screening for supportive care needs, written care plans and	✓ Australian Cancer Plan commitment to develop National Comprehensive Cancer Centre Network and models of care built on consistent use of MDTs ✓ Australian Cancer Plan commitment to develop performance framework for quality care with OCPs as the starting framework	Reflecting the benefits of a national approach to performance monitoring and costs of implementation, Cancer Australia is well placed to lead this effort but the Leukaemia Foundation should ensure the performance framework meets the needs of blood cancer patients. • Lead: Cancer Australia • Partners: Leukaemia Foundation and blood cancer community

Summary of actions	Progress to date	Implementation considerations
Improve referrals and equitable access to cancer-patient friendly exercise programs	✓ National Action Plan endorsement (Action 2.5) ✓ Multiple research funding proposals	Continue to work with and advocate to governments and wider NGO community to advocate for expansion of exercise oncology programs.
Policy reforms to reduce risks of financial toxicity	✓ National Action Plan endorsement ✓ Australian Cancer Plan commitments to focus on needs of priority populations (disadvantaged communities)	Continue to work with and advocate to governments and wider NGO community to advocate for expansion of exercise oncology programs. Reflecting benefits to all cancers and advocacy agenda of the Cancer Council Australia, the Leukaemia Foundation and blood cancer community is well placed to partner and support this work. • Leads: Cancer Australia and Cancer Council Australia • Partners: Leukaemia Foundation and blood cancer community

Figure 3.10: Priorities in catalysing health services reform and progress to date



Note: Provides a summary of progress to date and priorities going forward. The following acronyms are used: Australian Cancer Plan (ACP), non-government organisations (NGOs) and Optimal Care Pathways (OCPs). Source: Insight Economics.

Chapter 4
Accelerate Research

Research in blood cancers has made tremendous strides in the past 15 years, with the successful development and application of therapies leading to significant improvements in survival. Combined, blood cancers have achieved among the most significant improvement in 5-year survival over the past 34 years of any cancer group.

While significant gains have been made, addressing projected mortality and morbidity from blood cancers requires increased and sustained investment in research. More than 60 per cent of mortality and morbidity from blood cancers is due to an inadequate understanding of the disease and consequent absence of innovative therapies with curative potential.

In essence, the vision set out in the National Action Plan for zero lives to be lost to blood cancer by 2035 is only achievable through research.

Since the launch of the State of the Nation in 2019, there has been some progress towards creating a unified vision for what Australia's expert blood cancer researcher workforce can contribute to global research efforts – a Research Roadmap is currently under development with funding committed. Meanwhile, the development of the first-ever Australian Cancer Plan will see an attempt to address cancer agnostic data issues faced by Australian researchers through the development of a national cancer data ecosystem. This presents an opportunity for addressing systematic data issues that limit opportunities to make new breakthroughs in understanding disease biology and treatment.

This chapter briefly reviews the current landscape for accelerating research in Australia, looks at actions undertaken and considers priorities for the next horizon.

4.1 Achievements to date

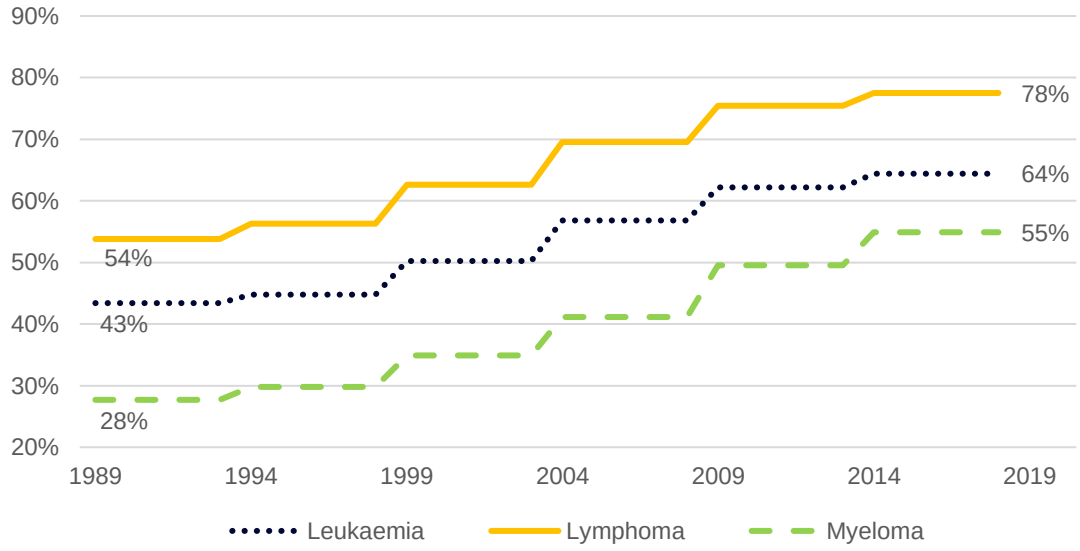
Research in blood cancers has made tremendous strides in the past 15 years, with therapies for paediatric cancers delivering cures for many children and the advent of tyrosine kinase inhibitor therapies enabling people with chronic myeloid leukaemia (CML) to live as long as their age-matched counterparts.

The successful development and application of therapies, enabled by long term support, has led to radical improvements in relative survival for blood cancer patients; for example, the Victorian Cancer Registry observed that blood cancers have faced the most significant improvement in five-year survival over the past 34 years (as seen in Figure 4.1):

> ***The most significant improvement in cancer 5-year survival over the past 34 years has been in haematological or blood cancers****, which include Hodgkin and non-Hodgkin lymphoma, leukaemia and multiple myeloma (also known as plasma cell myeloma). Since 1982-1986 lymphoma 5-year survival has increased from 49% to 79%, leukaemia from 40% to 70% and multiple myeloma from 26% to 63%. Improvement in 5-year survival for blood cancers is in large part due to discovery of novel targeted therapies.* [48]

[48] Victorian Cancer Registry, 2022, Cancer in Victoria 2021.

Figure 4.1: Changes in five-year relative survival rates for all Australians (1989-2018)



Source: AIHW and Cancer Council Australia.

However, while significant gains have been made, addressing projected mortality and morbidity from blood cancers as a collective is not possible without new discovery. The vision set out in the NAP for zero lives to be lost to blood cancer by 2035 is only achievable through research:[49]

> *More than 60 per cent of mortality and morbidity from blood cancers is due to an inadequate understanding of the disease and consequent absence of innovative therapies with curative potential. This major problem cannot be solved through the implementation of current best practice.* ***Investment in discovery and fundamentally, new treatment development is paramount.***

The 2019 SoTN also observed that there is limited funding and support for both fundamental research and investigator-led clinical trials in Australia. To address these challenges, the report called for more research funding.[50]

Drawing upon these findings and to provide a framework for action, the NAP recommendation the development of a Research Roadmap (Action 3.1.1, p.42).[51] The roadmap aims to build from Australia's role in world-leading research, as evidenced by work by the Centre of Excellence in CML, the development of the oral chemotherapy medication for blood cancer venetoclax, and the Zero Childhood Cancer Program, and citation analysis demonstrating the strength of Australian research in blood cancer (Box 4.1).

Box 4.1: Citation analysis shows strength of Australian research

Australian medical research is generally assumed to 'punch above its weight' in the evaluation of research impact. For example, in Cancer Institute NSW's evaluation of investment in cancer research it was estimated that Australian cancer researchers account for 2.5 per cent of total research impact (compared with a share of the world's population of only 0.3 per cent). Similarly, the NHMRC has recently estimated a contribution of 3.14 per cent (2008), 3.09 per cent (2013), and 3.6 per cent (2018) based on bibliometric analysis of medical research in its Measuring Up reports.

Scopus citation data for the 2010-2020 period show that over the past 10 years the impact of Australia's blood cancer community has consistently delivered a higher impact by a factor of two.

Australian researchers in blood cancer are pioneering in both research and clinical trials, making up an overweight share of clinical trials in blood cancer globally even in the context of Australia's small

[49] Leukaemia Foundation, 2020, National Strategic Action Plan for Blood Cancer.
[50] Leukaemia Foundation, 2019, State of the Nation: Blood Cancer in Australia.
[51] Leukaemia Foundation, 2020, National Strategic Action Plan for Blood Cancer.

population representing only 0.3 per cent of the world's population and around one per cent of reported blood cancer globally.

Australian share of publications, including high impact publications, 2010-2020

Citation analysis	All Australian medical research	Leukaemia	Lymphoma	Myeloma
Share of citations - all publications	3.1%-3.6%	6.3%	6.3%	5.2%
Share of citations - top quartile of publications		6.7%	5.6%	5.5%
Share of citations - publications with impact factor >10 (Top 2% of publications)		5.5%	5.8%	4.7%

Australian share of global clinical trials, 2000-2020 and 2015-2020

Blood cancer	Share of global population	2000-2020	2015-2020
Leukaemia	1.3%	8%	11%
Lymphoma	1.3%	9%	11%
Myeloma	0.9%	3%	10%

The NAP also noted that the number of identified blood cancer subtypes is increasing as understanding of the genetic basis of blood cancer improves. Paired with a small population, "splitting" into smaller subtypes frustrates Australia's ability to build adequate power to contribute to research efforts. It recommended that, to help address this challenge, Australia improve value and use of real-world data for blood cancer patients (Action 3.2 in the NAP, p.42).

Major achievements since the first SoTN in 2019 include:

- The Leukemia Foundation is leading the development of a Research Roadmap (current project)
- The Leukemia Foundation is supporting an Aboriginal and Torres Strait Islander Epidemiological Study (current project)
- The Leukemia Foundation continued to support Australian research, having committed $57.8 million to research since 2000
- The draft ACP proposed to develop a National Cancer Data Ecosystem.

Funding and development of a Research Roadmap

The Leukaemia Foundation has committed to funding the development of a Research Roadmap. The Research Roadmap, which is currently under development, will cover areas including blood cancer biology, genomics, microbiota, epidemiology, diagnostics, immunotherapies, targeted therapies and cellular therapies. It is expected that the Research Roadmap will be published in 2023.

Support for an epidemiological study of blood cancers in Aboriginal and Torres Strait Islander communities

In March 2022, the Leukaemia Foundation and the Federal Government entered a public-private partnerships to continue to fund actions outlined in the NAP. As part of that partnership, the Leukaemia Foundation committed to fund the first stage of the epidemiological study of blood cancer in Aboriginal and Torres Strait Islander populations.

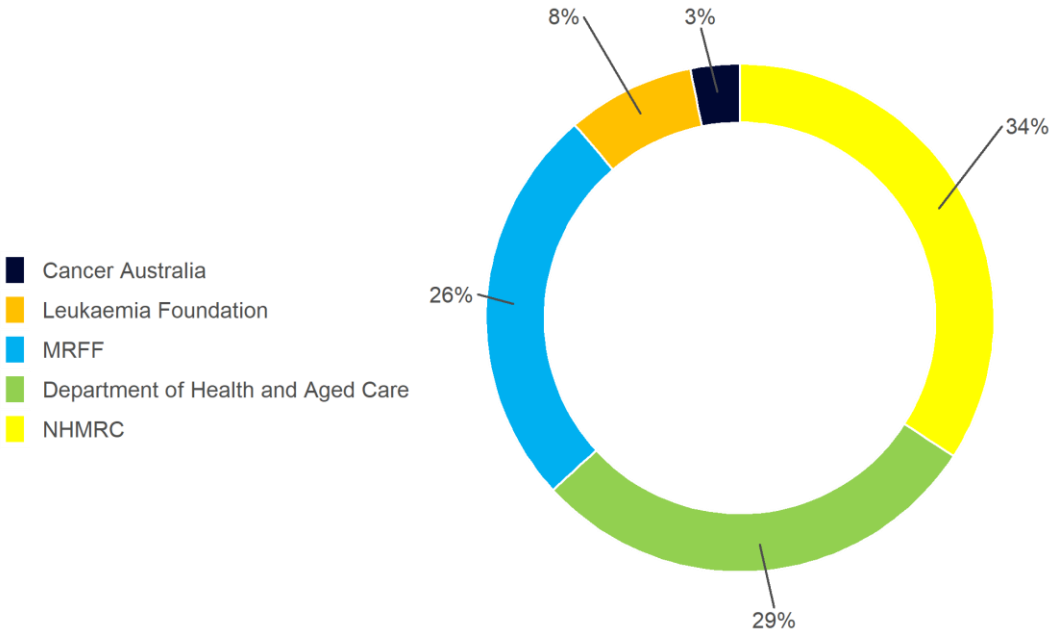
The project, which is under development, is being led by Professor Alex Brown, a leading Aboriginal researcher in partnership with leading research organisations across Australia. Currently, there is no expected publish data for phase one.

Support for research

Blood cancer research is funded from several key sources, including government (NHMRC, MRFF, Department of Health and Cancer Australia) and the not-for-profit sector (including the Leukaemia Foundation and Snowdome Foundation).

Over the period spanning 2019-2021, the Leukaemia Foundation provided more funding support for blood cancer research than Cancer Australia (Figure 4.2).

Figure 4.2: Funding share, key sources, 2019-2021



Note: Based on publicly available data. Source: Leukaemia Foundation, Grant Connect, MRFF and NHMRC, 2019-2021.

The Leukaemia Foundation has provided data to 2022, which is not publicly available for other listed data sources. Over the 2019-2022 horizon, the Leukaemia Foundation funded $11.9 million in blood cancer research, supporting key studies such as:

- The CAST study funded at the Centre for Blood Transplant and Cell Therapies at Westmead Hospital focused on the prevention of graft versus host disease (GVHD), a complication of allogeneic stem cell transplants
- Translational research for the development of next generation sequencing technologies at the SA Genomics Health Alliance
- The MOST-lly Blood Cancer Genomics trial as part of the National Genome Cancer Medicine Program
- A national, investigator-led clinical trial to optimise treatment for people with advanced Hodgkin lymphoma.

National cancer data ecosystem proposed in Australian Cancer Plan

The draft ACP has called for the development of a national cancer data ecosystem once launched in 2023. This includes goals for:

- In two years from launch, develop an agreed national cancer data framework to improve accessibility, consistency and comprehensiveness of integrated data assets.
- In five years from launch, the operationalisation of the national cancer data ecosystem supporting evidence-based innovation in models of care.

This will underpin performance reporting against OCP metrics of quality and safety and build an evidence base supporting and informing evidence-based, innovative models of care.

4.2 Key areas of need

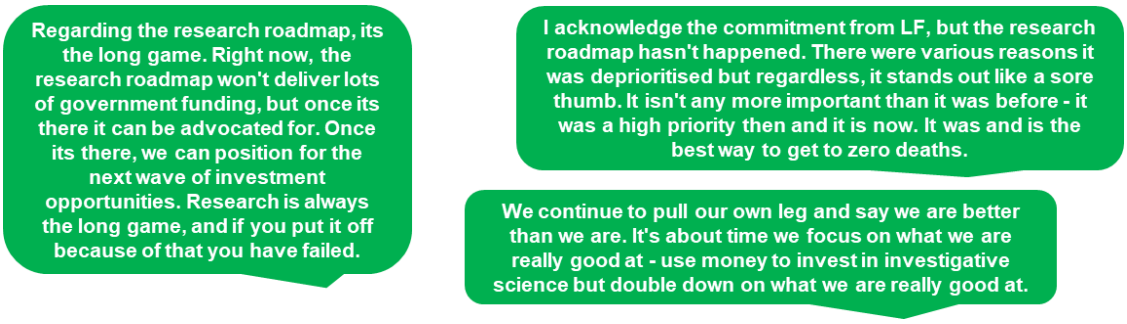
While Australia has made some progress towards accelerating research, more work is needed to unlock the potential of major scientific advances in curative therapies, such as CAR T-cell therapies, oncolytic viral therapies, clustered regularly interspaced short palindromic repeats (CRISPR) technologies that enable the editing of genes, targeted cancer drugs such as poly-ADP ribose polymerase (PARP) inhibitors, and preventative therapies. Key areas of need include:

- Completion of the Research Roadmap
- Stable and adequate funding of Australian research, including basic and translational research
- A national data ecosystem that integrates with international data.

Completion of research roadmap

The development of a Research Roadmap is a high priority and has only just been funded by the Leukaemia Foundation. Researchers and clinicians noted that, while complications have led to delays in its commissioning, the Research Roadmap remains critical and is in development (Figure 4.3).

Figure 4.3: Stakeholder perspectives on importance of Research Roadmap



Source: Insight Economics consultation with stakeholders, conducted in late 2022.

Key areas of research identified by patients and members of the blood cancer community include (Figure 4.4):

- Understanding what causes blood cancer
- Developing new models of care and evidence for supportive care
- Developing evidence for PROs.

The Research Roadmap will be focused on identifying Australia's relative areas of advantage in contributing to high impact, global research.

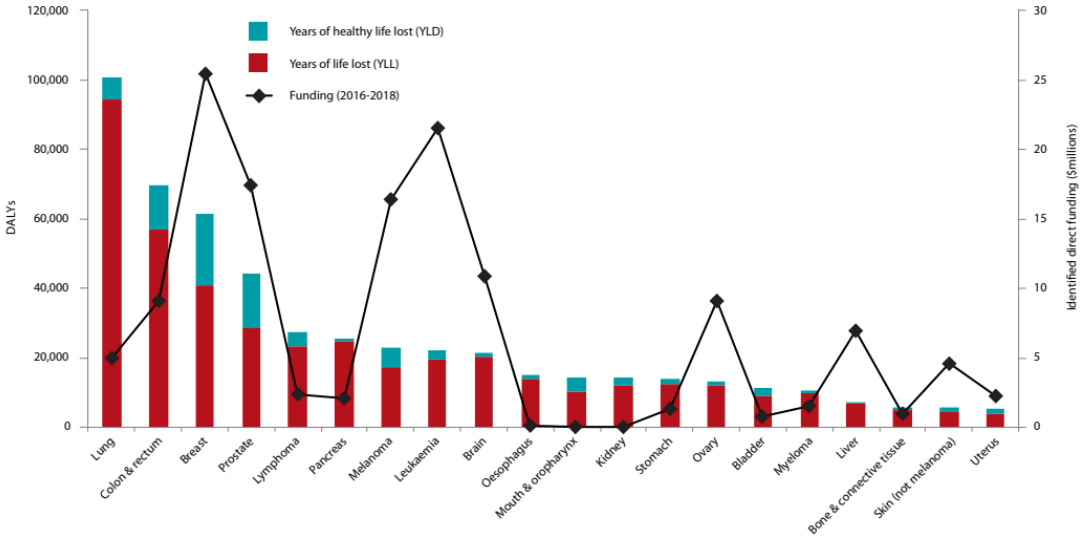
Figure 4.4: Perspectives on direction of Research Roadmap

Source: Insight Economics consultation with stakeholders, conducted in late 2022.

Increased and balanced funding for Australian blood cancer research

Although Australian researchers have demonstrated their propensity to undertake high quality research, blood cancer research is relatively underfunded. For example, Cancer Australia's 2016 review of funding indicates that, while leukaemia received relatively strong funding support over the period spanning 2016-2018, both lymphoma and myeloma received relatively low funding support (Figure 4.5).

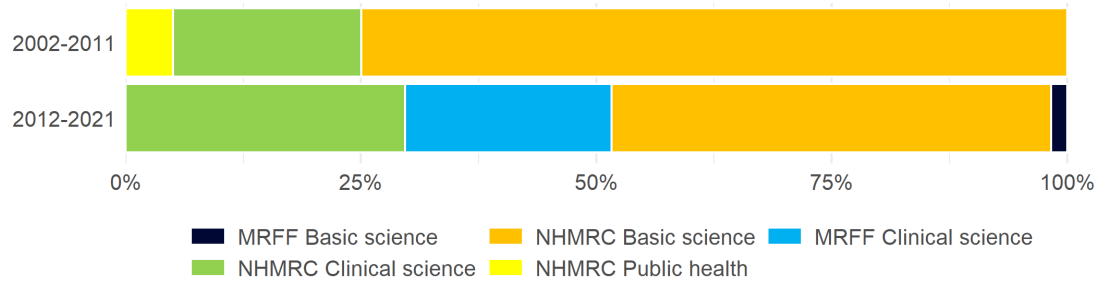
Figure 4.5: Research funding compared with burden of disease



Source: Cancer Australia, 2016, *Cancer Research in Australia 2016 to 2018 – Opportunities for strategic research investment*.

Despite the importance of enhancing our understanding of disease biology, the proportion of funding available for basic research from government continues to decline. Over the period from 2002-2011 basic research accounted for 75 per cent of all blood cancer funding; this contrasts to basic research's share of only 48 per cent of funding over the 2012-2021 period.[52] This is consistent with a broader decline in funding for basic research by the NHRMC, which has reduced funding for basic research across all cancers from 61 per cent to 53 per cent of funding for cancer research. Similarly funding for basic science across all medical research has declined from 48 per cent to 41 per cent (Figure 4.6).

Figure 4.6: NHMRC and MRFF funding for clinical and basic sciences, blood cancers

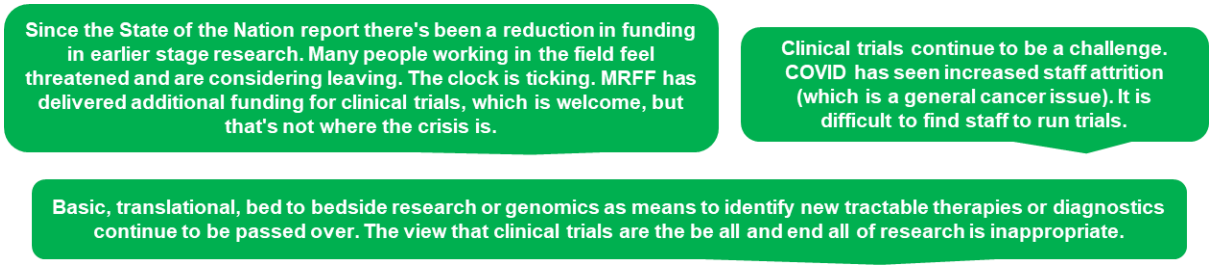


Note: Data filtered to contain the following: Leukaemia, Lymphoma, Myeloma, Myelodysplastic syndromes, Myeloproliferative neoplasms, Amyloidosis, Aplastic anaemia, Childhood blood cancers, Blood cancers. In addition, the following grants were excluded due to limited relevance: GNT1194811, GNT1177784, GNT1002473, GNT0571073, GNT0423405 and GNT038415.
Source: National Health and Medical Research Council and Medical Research Future Fund.

[52] This is based on MRFF and NHMRC data. In 2002-2011, the NHMRC provided $98 million in funding for basic science research. In 2012-2021, the NHRMC provided $90 million in funding for basic science; the MRFF provided $3 million for basic science research. Figures are blood cancers specific.

Declining funding for research may see Australia's propensity to contribute to blood cancer research diminish. Clinicians and researchers indicated that limited funding for research is contributing to an existential issue for Australian researchers, particularly in basic science and early phase research (Figure 4.7). In addition, attrition of the clinical trials workforce was observed to be impacting on ability to conduct research.

Figure 4.7: Stakeholder perspectives on impact of research workforce attrition, lack of support



Source: Insight Economics consultation with stakeholders, conducted in late 2022.

Ensuring national data ecosystem integrates with international data

As highlighted in the NAP, Australia must improve the value and use of real-world data for blood cancer patients. While members of the blood cancer community commended the draft ACP for its focus on improving the Australian Cancer data ecosystem, several points of interest and concern were raised (Figure 4.8):

- Nothing has yet been implemented, compared with foreign countries which are progressing policy
- The national data ecosystem must integrate with international data/research, otherwise it will not meet the needs of blood cancer research
- Patients require opportunities to contribute data including natural history data and PROs.

Figure 4.8: Stakeholder and patient comments on data

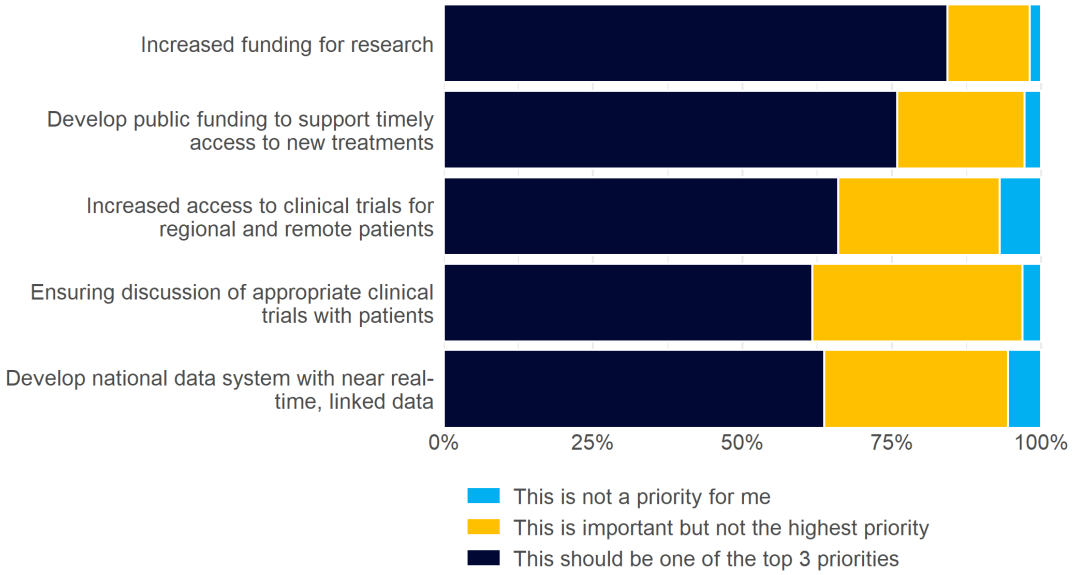
Source: Insight Economics consultation with stakeholders, conducted in late 2022.

4.3 Patient perspectives on priorities for accelerating research

Accelerating research was the second most commonly identified priority area by patients. This reflects that while improving the health system delivers benefits for patients today, achieving a cure requires new discovery.

Within the research strategic objective, the highest priority for patients was increasing funding for research, including public funding to support access to novel therapies, such as through a Right to Trial Fund and through increased access to clinical trials.

Figure 4.9: Ranking of strategic objectives



Note: Ranked from top (highest priority on average) to bottom (lowest priority on average). Source: People Living with Blood Cancer Survey 2022. See Appendix B.

4.4 Next horizon priorities

To promote optimal use of research dollars and galvanise investment, the Leukaemia Foundation and Blood Cancer Taskforce have a strong leadership role to play in the delivery of the Research Roadmap. Following delivery, the Leukaemia Foundation, Blood Cancer Taskforce, blood cancer community and state and federal governments including NHMRC and MRFF, should partner to fund the implementation of the Research Roadmap.

While the Research Roadmap is underdevelopment, the Leukaemia Foundation continues to support Australian research, through its support of an epidemiological study of blood cancer in Aboriginal and Torres Strait Islander communities.

Acknowledging the proposed development of a national cancer data ecosystem as part of the draft ACP, the Leukaemia Foundation will need to work in partnership with Cancer Australia to ensure the frameworks are designed to meet the needs of blood cancer patients and allow for the collection and aggregation of natural history data as well as clinical data. Reflecting small sample sizes, effort must be made to ensure these data can be exported and integrated with international data collection efforts (e.g., the HARMONY BigData Platform offering a central database and state-of-the-art Big Data analytical services designed to accelerate the development of more effective treatments for people with blood cancer)[53].

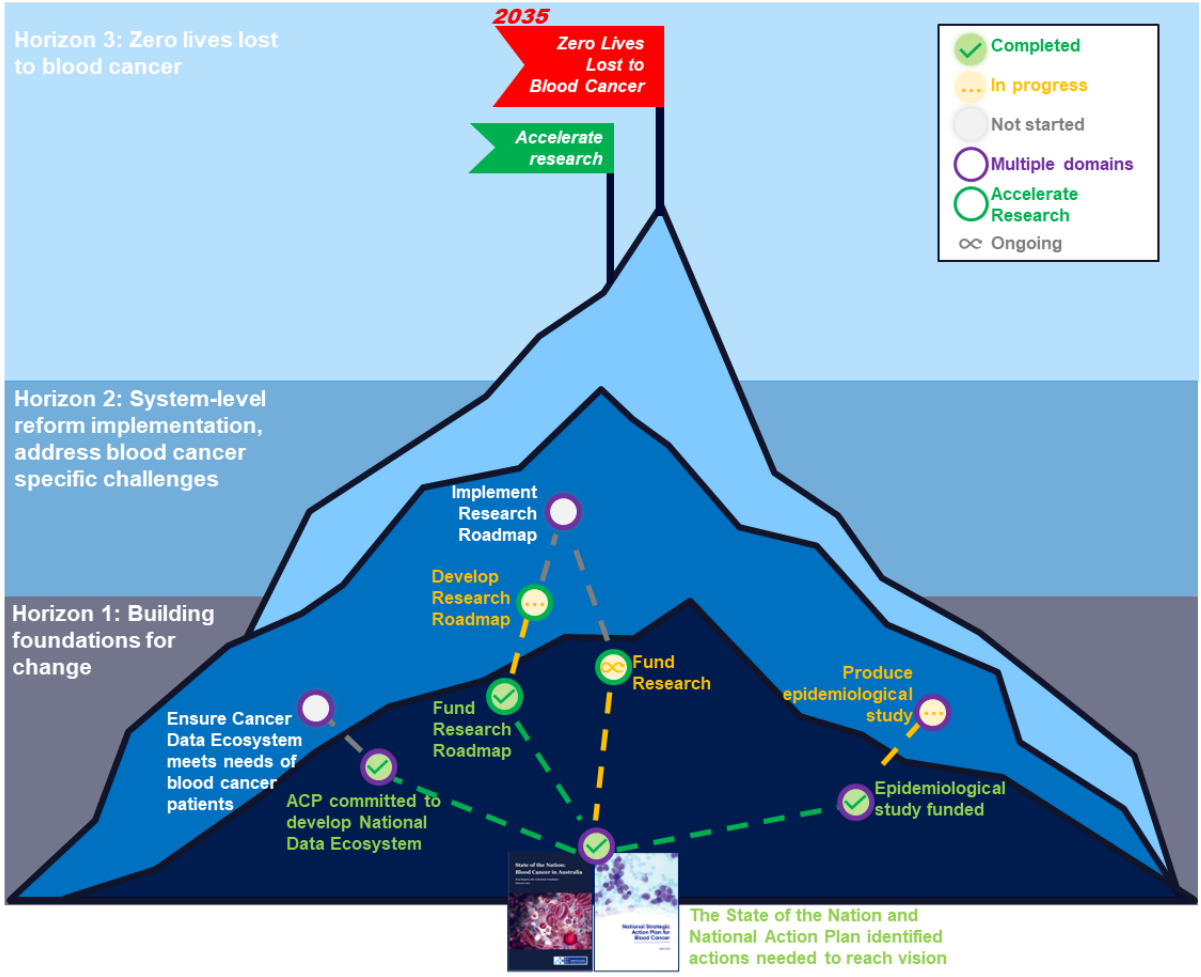
[53] Harmony Alliance, accessible at: https://www.harmony-alliance.eu/bigdata-platform/big-data-platform.

A summary of actions progressed to date and implementation considerations is presented in Table 4.1 and Figure 4.10.

Table 4.1: Summary of actions, progress to date and implementation considerations

Summary of actions	Progress to date	Implementation considerations
Identify priority areas for research funding that deliver greater impact through a Blood Cancer Research Program	✓ National Action Plan endorsement (Action 3.1) ✓ Leukaemia Foundation is supporting a Research Roadmap ✓ Leukaemia Foundation is supporting an Aboriginal and Torres Strait Islander Epidemiological study ✓ The Leukemia Foundation continues to support Australian research	Research Roadmap to be completed in 2023. Sustained funding including for basic science research will be needed to support successful research. • Lead: National Blood Cancer Taskforce • Partners: Australian Governments and blood cancer community
Improve value and use of real-world data for blood cancer patients	✓ National Action Plan endorsement (Action 3.2) ✓ Commitment within Australian Cancer Plan to development of a national cancer data ecosystem	Reflecting benefits to all cancers from system-level approach and need for capture of major cancer-specific needs • Lead: Cancer Australia • Partners: Leukaemia Foundation and blood cancer community

Figure 4.10: Priorities in accelerating research and progress to date



Note: Provides a summary of progress to date and priorities going forward. The following acronyms are used: Australian Cancer Plan (ACP). Source: Insight Economics.

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Chapter 5
Enable Access to Novel Therapies

Remarkable new advances in genomics, targeted therapies, immunotherapies, and other technologies are making the prospect of zero lives lost to blood cancer more real every day.

Realising the vision of zero lives lost to blood cancer by 2035 will require equitable and timely access to novel therapies. Blood cancer research is delivering significant innovation across a range of treatments, including novel immunotherapies, targeted therapies and breakthrough cellular therapies. Evidence suggests there is inconsistent access to novel therapies across Australia, which contributes to loss of life, poorer quality of life, and inequitable outcomes across patient groups that could be avoided through improvements to Australia's regulatory processes and systems for health funding.

Since the launch of the State of the Nation in 2019, governments have significantly improved their understanding of barriers to access and acknowledged that reforms are needed to address these barriers. While reform reviews are still ongoing, this is a major achievement of the past four years and the Leukaemia Foundation, working in partnership with the wider blood cancer community, has a major opportunity to see generational, step-change improvements in access arrangements for novel therapies in blood cancer.

5.1 Achievements to date

Since the launch of the first SoTN in 2019, there have been several positive improvements in access to novel therapies; these include:

- Approval and funding for 19 new therapies, including three CAR T-cell therapies
- Acknowledgement of the need for regulatory and funding model reform to improve access to therapies, as supported by:
  - The Federal Government's endorsement of the recommendations within the NAP
  - The recommendations of the House of Representatives Standing Committee on Health, Aged Care and Sport's Inquiry into approval processes for new drugs and novel medical technologies in Australia (the Zimmerman Report)
  - The National Long Term Health Reform Agreement 2022-2027
  - The initiation of the Health Technology Assessment review.
- Increased funding for clinical trials and clinical trials infrastructure.

Funding for new therapies

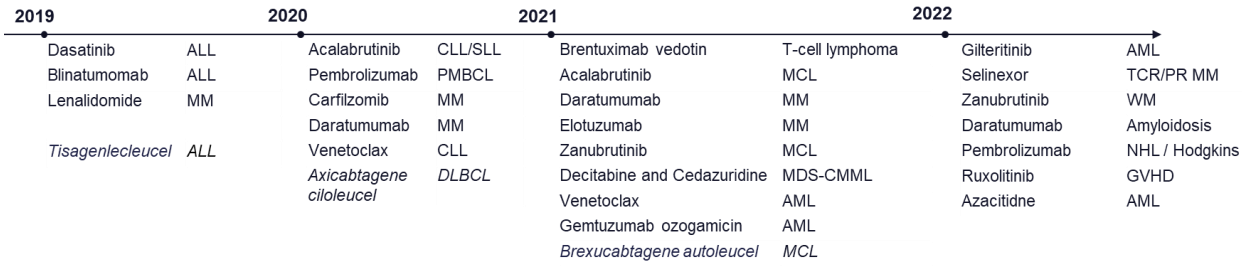
The first SoTN called for an improvement in access to blood cancer treatments to bring Australia into line with international best practice. Since that time, the Pharmaceutical Benefits Advisory Committee (PBAC) has made more than 16 positive PBAC recommendations for PBS listing of blood cancer treatments. This has included nine leukaemia therapies, six lymphoma therapies and seven myeloma therapies (Figure 5.1). Added to this, the Federal Government committed $80 million in funding for CAR T-cell

therapy manufacture and development of a CAR-T Centre of Excellence[54] in Victoria in 2019, with three therapies approved for use to date:

- *Tisagenlecleucel* – approved for paediatric and adolescent acute lymphoblatic leukaemia (ALL) (post-transplant) (<26 years) or in second or later relapse; adult relapsed or refractory diffuse large B cell lymphoma (DLBCL) after two or more lines of systemic therapy
- *Axicabtagene ciloleucel* – approved for adult relapsed or refractory large B-cell lymphoma after two lines of therapy
- *Brexucabtagene autoleucel* – approved for adult relapsed or refractory mantle cell lymphoma (MCL), who have received two or more lines of therapy, including a Bruton's tyrosine kinase (BTK) inhibitor (unless ineligible or intolerant to treatment with a BTK inhibitor).[55]

The Leukaemia Foundation and Blood Cancer Taskforce have been key stakeholders to the regulatory evaluation process for these new therapies since the launch of the report.

Figure 5.1: Listings of blood cancer therapies



Source: Leukaemia Foundation and Insight Economics analysis of PBAC outcomes and CAR T-cell therapy approvals, 2022, accessed at: https://www.pbs.gov.au/info/industry/listing/elements/pbac-meetings/pbac-outcomes.

Acknowledgement of the need for regulatory and funding reform

A major focus of the first SoTN was to raise awareness and propose solutions to address regulatory and funding barriers to access novel therapies. The report called for systemic reforms to Australia's regulatory systems and funding models to improve access. Government has responded to these calls for action by initiating a number of reviews and reform agendas. Specifically, the increased awareness and acknowledgement by government of a need for regulatory and funding model reform has been evident in:

- *The Federal Government's endorsement of the National Strategic Action Plan for Blood Cancer (2020)*[56] — The NAP called for a new approach to and funding for the development of a short-list of clinically important medicines and diagnostics that do not have public subsidy and where there are market barriers to evidence development. This was aligned to the recommendation of the first SoTN. In line with SoTN recommendations, it also called for:
  - Government and the blood cancer community to coordinate an approach to evidence development for each therapy, which could include funding investigator-led clinical trials, or coordination of research and regulatory applications, including provisional registration. This may require participation in a registry to enable access to a novel therapy.

[54] The CAR-T Centre of Excellence in Victoria including pre-clinical, translational and clinical services.
[55] Brexucabtagene autoleucel has marketing approval but funding agreements are not yet in place.
[56] Leukaemia Foundation, 2020, National Strategic Action Plan for Blood Cancer.

  - Government to commission a review of access to novel and specialised therapies by state and territory, in order to identify disparities in access to standard of care therapies and develop a plan to improve equity of access
  - Government to develop a strategy to optimise supply of suitable stem cell donors for Australian and international patients and to ensure equity of access to cellular and emerging therapies, including CAR T-cell therapy.

- *Zimmerman Report recommendations (2021)*[57] — The Zimmerman Report strongly supported the first SoTN and NAP recommendations, calling for the same reforms including a fund similar to the Right to Trial Fund. Key recommendations included:
  - Establish a Centre for Precision Medicine and Rare Diseases (CPMRD) within the Department of Health, which would include a comprehensive horizon scanning unit for new medicines and novel medical technologies (Recommendation 1)
  - Establish a clear and certain new Health Technology Assessment (HTA) pathway for cell and gene therapy (Recommendation 2)
  - Federal and state and territory governments to establish a jointly funded national genomics testing program to provide equitable access to genomic testing nationwide (Recommendation 2)
  - Federal Government to establish a fund to support patients, clinicians and non-profit organisations to sponsor registration and reimbursement applications where there is no realistic prospect of a company serving as sponsor, and where the Department of Health is otherwise supportive of the application (Recommendation 9)
  - Establish a new pathway that incentivises the repurposing of drugs (Recommendation 11).
- *The National Long Term Health Reform Agreement (2021)*[58] — The Agreement established long term reform streams in:
  - Nationally cohesive Health Technology Assessment, which includes the formation of a National High Cost and Highly Specialised Services Committee and a National High Cost Technology Assessment Framework to support coordinated and timely responses to new technologies by FY2023
  - Paying for Value and Outcomes, which includes a plan to develop a national health funding and payments framework by FY2023.
- *The National One Stop Shop and National Clinical Trials Front Door* (current) – Commissioned by the Federal Government, the Australian Commission on Safety and Quality in Health Care is developing a National One Stop Shop and National Clinical Trials Front Door aiming to make it easier for patients, researchers, industry representatives and sponsors to find, conduct, participate and invest in high quality and ethical research in Australia.[59] It will:

[57] Commonwealth Government, 2021, The New Frontier – Delivering better health for all Australians: Inquiry into approval processes for new drugs and novel medical technologies in Australia, House of Representatives Standing Committee on Health, Aged Care and Sport, accessed at: https://www.aph.gov.au/Parliamentary_Business/Committees/House/Health_Aged_Care_and_Sport/Newdrugs/Report.
[58] Department of Health and Ageing, 2021, National Health Reform Agreement (NHRA) – Long-term health reforms roadmap, accessed at: https://www.health.gov.au/resources/publications/national-health-reform-agreement-nhra-long-term-health-reforms-roadmap?language=en#:~:text=National%20Health%20Reform%20Agreement%20%28NHRA%29%20%E2%80%93%20Long-term%20health,and%20ensure%20our%20health%20system%20is%20sustainable.%20Print
[59] Australian Commission on Safety and Quality in Healthcare website, accessed at: https://www.safetyandquality.gov.au/our-work/health-and-human-research/national-one-stop-shop-national-platform-health-related-human-research.

  - Provide a cross-jurisdictional ethics approval and site-specific authorisation platform that incorporates key application, notification and approval systems
  - Incorporate the Clinical Trials Notification and Clinical Trials Approval schemes administered by the TGA
  - Include an embedded and automated next-generation national clinical trials registry
  - Provide sophisticated monitoring and reporting functionality for different users.
- *The Health Technology Assessment Review (current)* — In response to the Zimmerman Report recommendations and a *Strategic Agreement 2022-2027* between Medicines Australia and the Federal Government,[60] the Federal Government agreed to conduct an independent review of Australia's HTA system. This will be the first review of its kind in 30 years and is to be completed by July 2023. The scope of the review was defined in October 2022 to include:
  - Selection of comparator(s), i.e., therapies which are used to benchmark benefits from therapies and/or technologies
  - Methods for evaluating rare diseases for reimbursement and alternative funding pathways if required
  - Methods for evaluating new and emerging technologies (including cell and gene therapies, and other precision-based medicines) and the suitability of existing funding pathways as required
  - Methods for evaluating all new medicines and vaccines
  - Use of real-world evidence including sources other than randomised controlled trials
  - Managing clinical, economic, financial and other uncertainty
  - The feasibility of international work-sharing for reimbursement submissions.

Together, these major reform processes have the potential to open the door to the significant, system-level reforms called for in the first SoTN, which are needed to address the barriers to timely, equitable access to novel therapies. Of course, because these are large, multilateral systems-level reforms, they take time to implement; nonetheless, the catalyst of these reform initiatives represents a significant positive step for people living with blood cancer, generated by patient support advocacy.

Access to clinical trials, including tele-trials

Clinical trials are the tools through which evidence is developed to inform changes in clinical practice. Clinical trials are also an opportunity for patients to access the latest products, techniques and technologies, which have the potential to deliver quality of life improvements above standard therapies, along with increased rates of survival.

The NAP called for additional funding for clinical trials in blood cancer; an increase in the use of tele-trials to reduce access barriers to trials for rural and remote patients; and KPIs to foster a research-ready clinical practice environment. The 2019 Rural, Regional and Remote Clinical Trial Enabling Infrastructure grant opportunity provides between $5 million and $100 million for new and innovative approaches to enhance and improve clinical trial

[60] Medicines Australia, 2021, Medicines Australia Strategic Agreement 2022-2027, accessed at: https://www.medicinesaustralia.com.au/wp-content/uploads/sites/65/2021/09/Medicines-Australia-Strategic-Agreement-2022-2027.pdf.

infrastructure, and to extend existing clinical trials into regional and remote areas of Australia. The COVID-19 pandemic response, too, saw an expansion in the use of tele-trials.[61]

The One Stop Shop and National Clinical Trials Front Door proposal would also facilitate access through streamlining processes in establishing multi-site trials.

5.2 Key areas of need

While there have been 'green shoots' in terms of improving access for novel therapies, more work is needed to improve timely and consistent access to novel therapies in clinical practice and clinical trials. Key areas of need include:

- Implementation of recommendations for HTA reform and improving the harmonisation of evidence development, including:
  - Modernising approaches to evidence development and the management of uncertainty in light of increasing sophistication in the characterisation of disease through genomics, which has seen traditional models of evidence development upended over the past 20 years
  - Establishing a fund (a 'Right to Trial' program) to support evidence development for therapies where there limited market incentive for listing, such as for off-label use or repurposing medications
  - Developing a national policy for access to cellular and genetic therapies, which challenge Australia's regulatory and funding processes by consequence of their high cost and Federal-state funding challenges
- Genomic testing as the standard of care
- Improving the security of supply in cellular therapies
- Improving awareness of, and participation in, clinical trials as part of the development of a 'research-ready' culture in Australian clinical practice.

Implementation of HTA reform recommendations

Comparing current Australian clinical practice for diagnostics and international clinical guidelines for standards of care issued by the NCCN, ESMO, and NICE reveals that a number of therapies that are in use overseas are not used in routine clinical practice in Australian care settings (Table 5.1).

Table 5.1: Access gaps in blood cancer diagnosis and treatment

Sub-type	Diagnosis or monitoring testing	First line treatment options	Relapsed and refractory treatment options
ALL	Minimum Residual Disease testing and monitoring which allows for assessment of the depth of remission following therapy and optimise the use of allogeneic stem cell transplantation.	blinatumomab imatinib for Ph-like ALL nilotinib for Ph-like ALL dasatinib for Ph-like ALL ponatinib for Ph-like ALL	CAR T-cell therapy for adults (over 25) Lack of options for relapsed T-ALL

[61] Underhill, C., Parente, P., McArthur, G., et al., 2020, Victorian COVID-19 Cancer Network. Towards new models of cancer care in Australia: lessons from Victoria's response to the COVID-19 pandemic. Intern M, 50(10), 1282-1285, doi: 10.1111/imj.15012; Roberts, N.A., Cubitt, A., Lindsay, D., et al., 2022, Teletrials, the new norm? Expert recommendations for teletrials into the future: Findings from the Clinical Oncology Society of Australia Clinical Trial Research Professionals Group Workshop, Asia-Pac J Clin Oncol, 18, 650– 659, doi: 10.1111/ajco.13737.

Sub-type	Diagnosis or monitoring testing	First line treatment options	Relapsed and refractory treatment options
	Lack of funding options for advanced diagnostics (PCR, genomic array, NGS). As a result, several WHO defined ALL sub-entities are not routinely discoverable with current standard of care diagnostics, precluding selection of optimal therapy	PBS restrictions on TKI use beyond 2 years of maintenance	
AML	Lack of funding options for advanced diagnostics (MRD PCR and flow, microarray, NGS), which may impact on transplant decisions.	Many drugs are not funded for inpatient use despite being available on the PBS, which is restricted to outpatient medication use, with major examples including midostaurin, gemtuzumab ozogomycin, venetoclax-azacitidine. gliteritinib (*FLT3* inhibitor) IDH-1 and 2 inhibitors (enasidenib, ivosenib)	Certain MRD monitoring is not undertaken post diagnosis due to the lack of targeted treatment options for some of those markers if they were to relapse/progress. Cell therapy
Lower Risk MDS	Molecular testing unfunded/unavailable	Therapies that improve cytopaenias and /or transfusion dependence are largely unavailable or unfunded (e.g. EPO and luspatercept)	Therapies for relapsed / refractory MDS are nonfunded or unavailable
Intermediate and / or higher risk MDS	Molecular testing unfunded/unavailable and this may change risk and /or allotransplant decisions	Therapies for patients with 10-20% blasts are limited with CR rates<50% and /or significant toxicities for combination therapy (e.g., venetoclax)	Nonfunded or unavailable for MDS phenotype unless patient progresses to AML
CML	-	-	-
CLL	Access to gene array and mutation screening by NGS (e.g. TP53) Frontline young patients no access to novel agents Inequitable access of genetic testing necessary to inform prognosis	acalabrutinib ± obinutuzumab, venetoclax + obinutuzumab for younger patients zanubrutinib ibrutinib	CAR-T cell therapy Triple refractory population- gaps in drug options Richter's Transformation - gaps in drug options Access to venetoclax retreatment Double refractory treatment options pirtobrutinib
Lymphoma	Lack of funding options for advanced diagnostics (NGS	critzotinib (ALK+Lymphoma,	azacitidine (T-Cell)

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

Source: Review of latest available NCCN, ESMO and NICE clinical guidelines; Department of Health, 2022, Application 1684, Genetic testing for variants associated with haematological malignancies; and National Action Plan for Blood Cancer, Chapters 3 and 5.

Many novel diagnostics remain unfunded, even though they are used as the standard of care in Australia and overseas, as developing the required evidence base may be time-consuming, complex or expensive (or a combination of all three).

Access gaps have been further compounded by the advent of new, high-cost cellular therapies such as CAR T-cell therapies, which require a mix of state and federal funding and straddle traditional regulatory pathways, involving both product and service elements. Feedback is that the process for establishing new centres has been *ad hoc* and lacking in transparency, complicated by federated funding models in health and compounded by factors not related to quality or safety, but rather issues of cost-shifting and competition. Today, eight centres provide CAR T-cell therapy across Australia, but these are concentrated in NSW (4 centres), Victoria (2 centres) and Queensland (2 centres), with an unclear process for establishing sites. To date, funding for these services has been based on the National Health Reform Agreement (50% state and 50% Federal).

The need for reform has been acknowledged by government through multiple reviews, recommendations and reform initiatives underway. Three major reforms must be implemented from the proposed recommendations:

- Modernise evidence development and the management of uncertainty to improve timely access to novel therapies
- Harmonise evidence development, including through the establishment of a Right to Trial Fund
- Establish a pathway for cellular and genetic therapies.

These reforms are considered in turn.

Modernise access to novel therapies and the management of uncertainty

Australia's regulatory frameworks have traditionally required evidence of improvement in overall survival in Australian populations through the conduct of randomised controlled trials. This is, of course, consistent with best practice evidence-based medicine. Notwithstanding, as the costs of evidence development increase and blood cancers stratify into smaller patient populations, these traditional approaches to evidence development are challenged, and can result in issues including undesirable delays in access to clinically important therapies. There is increasing recognition that, to address potential issues in timely access to therapies, Australia's regulatory processes must evolve.[62]

The first SoTN, for example, highlighted the example of TKIs in ALL, where there were positive signals of benefit as early as 2013 for patients with BCR-AB1 mutations, but no TKI therapy was PBS-listed for ALL until 2018; more than 500 patients would have been diagnosed over the 2013-2018 period, and patients with AB1 mutations would have been likely to survive.[63]

The NAP also highlighted the use of crizotinib in ALK+ anaplastic large cell lymphoma in children. Anaplastic large cell lymphoma is a rare form of non-Hodgkin lymphoma (NHL) and accounts for approximately 30 per cent of NHL cases in young people, with approximately 90 per cent of anaplastic large cell lymphoma cases in paediatric patients.[64] In Australia, this translates to roughly 16 children and young adults diagnosed with ALK+ anaplastic large cell lymphoma each year. Crizotinib remains unlisted in Australia today, even though a Phase II study in 2017 showed overall response rates of 80 to 83 per cent for patients aged 1-21 years old, noting that many patients do not have any curative treatment options and experience relapse following chemotherapy.[65] In 2021, the US Food and Drug

[62] As manifest in the Zimmerman Report recommendations and the current HTA review.
[63] Weston, B.W., Hayden, M.A., Roberts, K.G., et al., 2013, Tyrosine Kinase Therapy Induces Remission in a Patient with Refractory EBF1-PDFRB-Positive Acute Lymphoblastic Leukaemia, Journal of Clinical Oncology, 31(25).
[64] Pfizer's Xalkori (Crizotinib) Approved By FDA For ALK-Positive Anaplastic Large Cell Lymphoma in Children and Young Adults [news release], Pfizer; January 14, 2021. https://investors.pfizer.com/investor-news/press-release-details/2021/Pfizers-XALKORI-crizotinib-Approved-by-FDA-for-ALK-positive-Anaplastic-Large-Cell-Lymphoma-in-Children-and-Young-Adults/default.aspx.
[65] Mosse, Y.P., Voss, S.D., Lim M.S., et al., 2017, Targeting ALK with Crizotinib in Pediatric Anaplastic Large Cell Lymphoma and Inflammatory Myofibroblastic Turmour: A Children's Oncology Group Study, J Clin Oncol, doi: 10.1200/JCO.2017.73.4830.

Administration (FDA) approved the use of crizotinib in ALK+ anaplastic large cell lymphoma in paediatric populations on the basis of this study.[66] Since 2017, more than 80 children and young adults have been diagnosed with ALK+ anaplastic large cell lymphoma that may have benefited from early access to this therapy.

Evidence of clinical benefit has also been observed and reported for other blood cancers, including the use of CAR T-cell therapies in diffuse large B-cell lymphoma and myeloma:

- Diffuse large B cell lymphoma is the most common subtype of non-Hodgkin lymphoma and is estimated to account for 30 per cent to 40 per cent of all new cases. In Australia, this translates to 2,000 to 2,700 new cases of diffuse large B-cell lymphoma each year. Of these patients, approximately 390-530 patients are expected to experience relapsed/refractory disease following treatment with curative intent, with 200-400 being refractory to treatment.[67] Response rates for patients with relapsed/refractory diffuse large B cell lymphoma are estimated to vary from 14 per cent to 63 per cent. For refractory patients, the outlook is particularly poor: treatment of refractory patients achieves a complete response (CR) rate of only eight per cent, with a median overall survival (OS) of 6.6 months, indicating a major unmet need for effective therapies for these patients. In 2017, a Phase 1 study found nine patients with refractory diffuse large B-cell lymphoma aged 34 to 69 treated with CAR T-cell therapy (axicabtagene ciloleucel) achieved a complete response (CR) rate of 57 per cent, with 30 per cent of patients in the study reporting ongoing CR (all at 12+ months). This regimen was found to be safe for further study in phase 2 and induced durable remissions in patients with refractory DLBCL. These response rates were confirmed in 2019, with a Phase I/II study of 118 patients achieving similar durability of response and a median overall survival of greater than two years. The data showed 58 per cent of patients achieved a complete response and that the median duration of response was more than 11 months.[68] A phase 3 study of axicabtagene ciloleucel as second line therapy for large B-cell lymphoma found that axi-cel therapy led to significant improvements at a median follow up of 24.9 months, with median event-free survival of 8.3 months compared to 2.0 months in the standard-care group.[69]
- Similarly, recent clinical trials of the use of CAR T-cell therapy in myeloma found that its use achieved a complete response rate of 67 per cent and an improvement of overall survival at one year of 89 per cent, which compares favourably to current average 1-year survival rates of 84.8 per cent in Australia today.[70] In 2022, approximately 2,700 Australians will have been diagnosed with myeloma; a four percentage point improvement in 1 year survival would translate into more than 110 additional Australians alive in a year than would have otherwise been the case.

These three case study examples alone identify more than 400 patients each year that could benefit from improving access to clinically important therapies.

[66] FDA, 2021, FDA approves crizotinib for children and young adults with relapsed or refractory, systemic anaplastic large cell lymphoma, accessed at: https://www.fda.gov/drugs/resources-information-approved-drugs/fda-approves-crizotinib-children-and-young-adults-relapsed-or-refractory-systemic-anaplastic-large.

[67] Harrysson, S., Eloranta, S., Ekberg, S., et al., 2021, Incidence of relapsed/refractory diffuse large B-cell lymphoma (DLBCL) including CNS relapse in a population-based cohort of 4243 patients in Sweden, Blood Cancer J, 11(9), doi: 10.1038/s41408-020-00403-1; Flowers, C.R., Odejide, O.O., 2022, Sequencing therapy in relapsed DLBCL, Hematology Am Soc Hematol Educ Program, 2022(1), 146–154, doi: 10.1182/hematology.2022000332.

[68] Locke, F.L., Ghobadi, A., Jacobson, C.A., et al., 2019, Long-term safety and activity of axicabtagene ciloleucel in refractory large B-cell lymphoma (ZUMA-1): a single-arm, multicentre, phase 1-2 trial, Lancet Oncol, 20(1), 31-42, doi: 10.1016/S1470-2045(18)30864-7.

[69] Locke, F.L., Miklos, D.B., Jacobson, C.A., 2022, Axicabtagene Ciloleucel as Second-Line Therapy for Large B-Cell Lymphoma, N Engl J Med 2022; 386:640-654, doi: 10.1056/NEJMoa2116133.

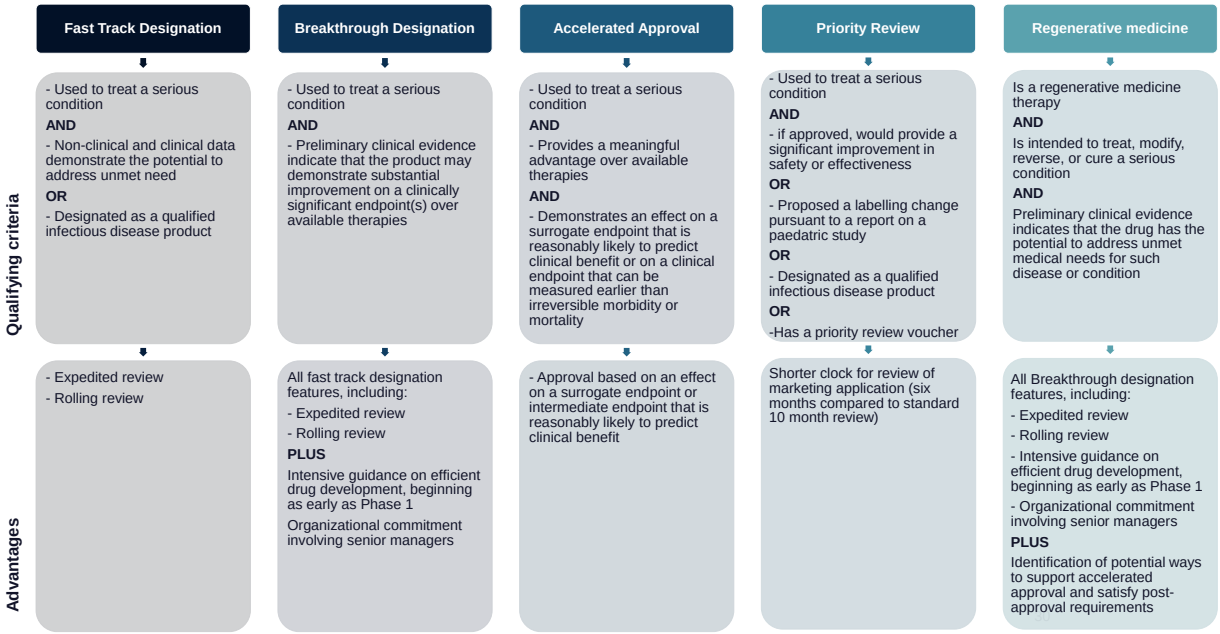
[70] Berdeja, J.G., Madduri, D., Usmani, S.Z., et al., 2021, Ciltacabtagene autoleucel, a B-cell maturation antigen-directed chimeric antigen receptor T-cell therapy in patients with relapsed or refractory multiple myeloma (CARTITUDE-1): a phase 1b/2 open-label study, Lancet, 398(10297), 314-324, doi: 10.1016/S0140-6736(21)00933-8.

Australia is not alone in confronting new approaches to evidence and uncertainty. The US has created four[71] programs to facilitate and expedite development and review of new drugs to address unmet medical need in the treatment of a serious or life-threatening condition: fast track designation, breakthrough therapy designation, accelerated approval, and priority review designation (see section IV for an overview of the programs).[72] Recently, it has also added a Regenerative Medicines for Serious Conditions pathway. Through an ongoing regulatory review process, it has:

- Defined a serious condition and unmet medical need
- Defined a regenerative medicine, with further classifications based on whether the medicine is intended to treat, modify, reverse or cure a serious condition
- Established criteria to apply for fast track, breakthrough, accelerated approval, and priority review designations
- Identified accepted preliminary clinical evidence and accelerated approval endpoints.

The major qualifying criteria and features of each pathway are shown in Figure 5.2.

Figure 5.2: FDA approaches to access and uncertainty



Source: FDA, 2022, Expedited Programs for Regenerative Medicine Therapies for Serious Conditions: Guidance for Industry, accessed at http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm; and FDA, 2020, Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics, accessed at: https://www.fda.gov/media/86377/download.

Regulatory bodies in Europe and the UK have similarly adapted their regulatory pathways to accommodate increasing uncertainty and technological change:[73]

- The European Medicine Agency (EMA) established a conditional marketing authorisation, which similarly is available for products with unmet medical need, for

[71] Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics, May 2014, available at https://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm358301.pdf; FDA, 2020, Guidance for Industry and FDA Staff: FDA Acceptance of Foreign Clinical Studies not conducted under an IND Frequently Asked Questions, available at https://www.fda.gov/downloads/RegulatoryInformation/Guidances/UCM294729.pdf.

[72] FDA, 2020, Guidance for Industry, Expedited Programs for Serious Conditions – Drugs and Biologics, https://www.fda.gov/media/86377/download.

[73] Crane, R., 2021, European Expedited Regulatory Programs Explained, accessed at: https://www.propharmagroup.com/blog/european-expedited-regulatory-programs/

seriously debilitating and life-threatening disease, where there is a lack of available treatment or a major improvement over existing therapy. Conditional marketing authorisations can be approved on the basis of less-complete clinical data (Phase 2 uncontrolled trials), but there must be a demonstration of a positive benefit-risk balance at the time of filing and a plan for comprehensive data to be submitted post-approval using a confirmatory trial. The EMA also offers a PRIority MEdicines (PRIME) scheme, which is similar to the FDA's Breakthrough Designation, and provides for approvals based on earlier clinical data than is required for a conditional marketing authorisation. Products are assessed by an expert committee, the Rapporteurs, which are reviewers selected from EU members states and evaluate early data.

- In the UK (which recently established the Medicines and Healthcare products Regulatory Agency, MHRA), new pathways have been developed to accelerate access to novel therapies. The UK offers conditional marketing authorisations on the basis of less-complete clinical data (Phase 2 uncontrolled trials) and in January 2021, the UK launched the Innovative Licensing and Access Pathway (ILAP). Through the ILAP, the MHRA will help the Sponsor (developer of medicine, UK or global, both commercial and non commercial) develop the target product profile and roadmap to approval and will accept the clinical data for MAA evaluation under a rolling review. The UK has joined Project Orbis, where it is able to coordinate with the US, Canada and Australia in reviewing evidence for and approving promising cancer drugs.

  At the same time, the UK's National Institute for Health and Care Excellence (NICE) has also revised its health technologies assessment processes, to:[74]

  - Apply a disease severity modifier to health technology evaluations to give more weight to health benefits in the most severe conditions
  - Clarify analysis of uncertainty
  - Allow more flexibility for NICE's independent committees in cases when generating evidence is difficult
  - Broaden the consideration of evidence to include real-world evidence, health-related quality of life, and costs
  - Develop a vision statement, principles and criteria for the assessment of treatments for very rare diseases in the Highly Specialised Technologies (HST) Programme, which includes recommendations on the use of new and existing highly specialised medicines and treatments
  - Align evaluation processes across medicines, medical technologies and diagnostics pathways.

Australia's PBAC and Medical Services Advisory Committee (MSAC) regulatory authorities are not directly comparable to the FDA, EMA or MHRA, which are more analogous to Australia's TGA. Nevertheless, as Australia has modernised its Therapeutic Good Administration (TGA) pathways the HTA review provides the opportunity to similarly modernise its reimbursement pathways. Australia should develop additional reimbursement pathways that allow for the use of earlier and more varied clinical data, including international data such as through Project Orbis, coupled with rolling review of confirmatory clinical trials or real world data. For example, PBAC could consider and recommend reimbursement for a product based on early results, such as the ZUMA-1 or CARTITUDE-1

[74] NICE, 2022, Review of methods, processes and topic selection for health technology evaluation programmes: conclusions and final update, Appendix: Further discussion and rationale for conclusions – methods, accessed at: https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation.

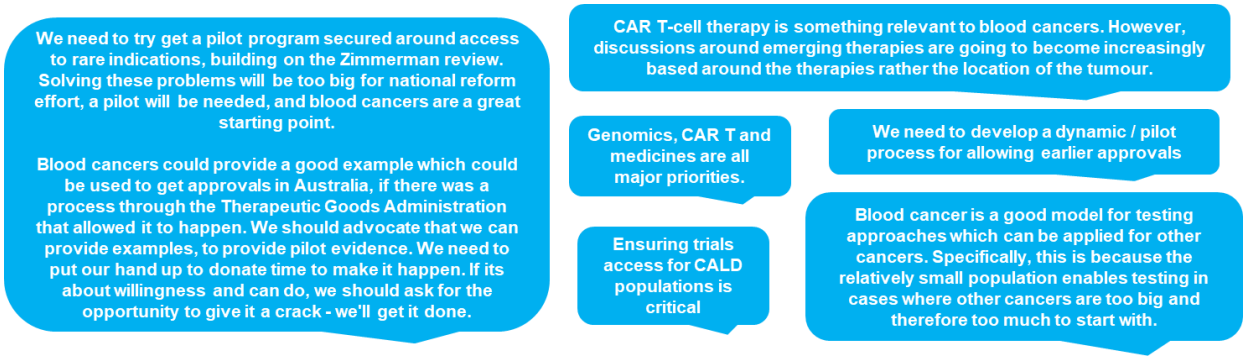
study, and then revise the rate of reimbursement based on updated results. Australia should also continue to expand its use of international data and real-world evidence.

While Australia has a managed entry scheme available to support coverage with evidence development, in practice it is not used; greater engagement with industry is needed to improve the use of conditional approvals to support early listings. Rate of reimbursement should be a function of the levels of evidence available to support listing.

Harmonise evidence development

In addition to broadening the scope of evidence and pathways for reimbursement, government should also work to support evidence development for clinically important diagnostics and therapies. This was consistently identified as a major area of need by members of the blood cancer community (Figure 5.3).

Figure 5.3: Stakeholder perspectives on areas of need to enable access to novel and specialised diagnostics and therapies



Source: Insight Economics consultation with stakeholders, conducted in late 2022.

This would involve systematic identification of therapies and/or diagnostics where there are signals of clinical benefit and/or where these are recommended by Australian or international clinical guidelines but there may not be a commercial incentive for industry submission or there is insufficient coordination among clinicians.

To harmonise evidence development, regulatory approvals, and service development, the NAP called for the establishment of a blood cancer working group to undertake horizon scanning and coordinate research activities across the sector in order to facilitate evidence development for regulatory approval and funding for clinically important diagnostics, medicines and services.

The Zimmerman Report endorsed such an idea but, similar to the draft ACP, broadened the scope of the reform beyond blood cancers alone. Specifically, the Zimmerman Report recommended the creation and funding of a CPMRD within the Department of Health to undertake a broad program of work, including:

- Horizon scanning for clinically important therapies
- The coordination of evidence development through investigator-led clinical trials and real world data, consistent with international approaches such as the INFORMED, CancerLinQ and Project Orbis programs
- Increasing access to new medicines through additional regulatory and reimbursement pathways, including the increased use of coverage with evidence development (or managed entry scheme) programs for clinically important therapies and the establishment of a Right to Trial Fund.

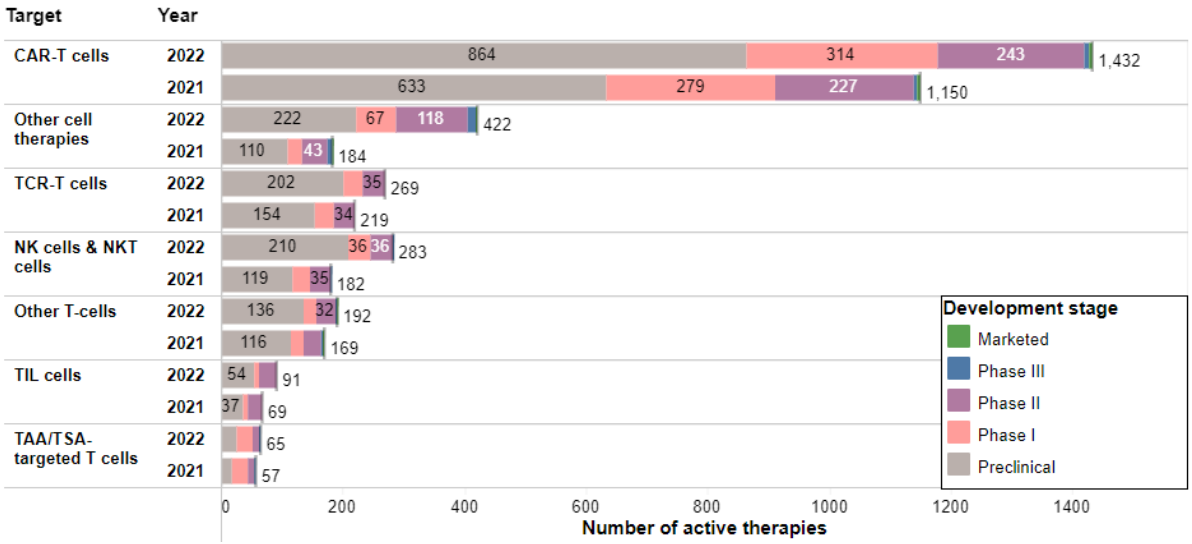
The Blood Cancer Taskforce should evolve to be an expert reference group to the CPMRD, as the peak consumer and clinical body with whom the CPMRD could engage on the specific needs and therapies for blood cancer patients going forward.

A harmonised, national approach to cellular and genetic therapies

The first order of business for the CPMRD should also be a national policy for the commissioning, funding and governance of CAR T-cell and other cellular and genetic therapies, which are entering mainstream clinical practice.

While over time CAR T-cell and other cellular therapies will extend to other cancers, blood cancers are leading the way in the use of these services. The Cancer Research Institute estimates more than 1,432 CAR T-cell therapies are in development in aggregate, with more than 464 targeting blood cancers alone (Figure 5.4).

Figure 5.4: There is a huge wave of CAR T-cell therapies on the horizon

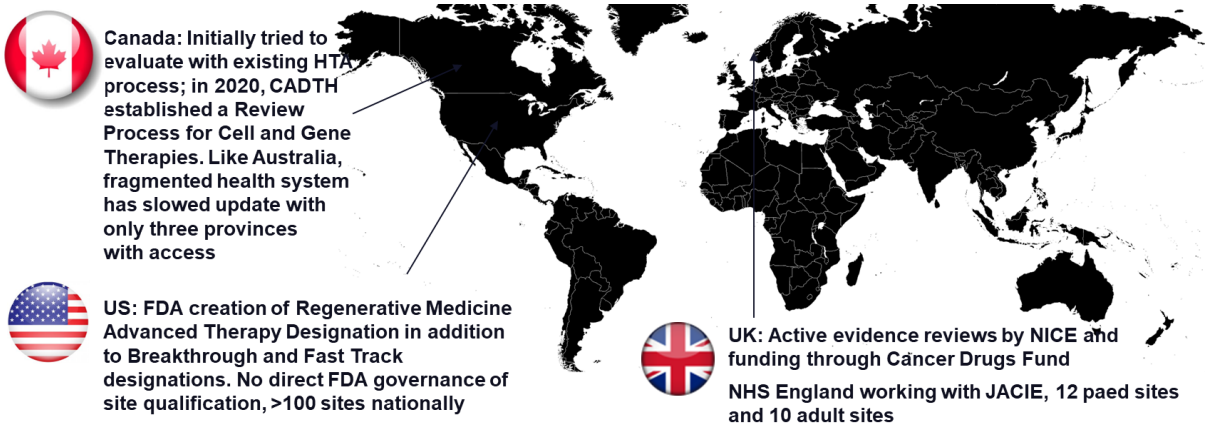


Source: Nature Reviews Drug Discovery, June 2022, accessed at: https://www.cancerresearch.org/cancer-cell-therapy-landscape.

Improving the transparency and timeliness of CAR T-cell therapies will be critical to improving blood cancer survival. The advent of CAR T-cell therapies represents a major advance in immunotherapies, holding the promise of a potentially curative therapy for patients with a low survival outlook. Blood cancer patients, clinicians, and governments alike will benefit from a coordinated strategy for the commissioning of these services.

In developing a national approach to CAR T-cell therapies, Australia will bring itself in line with international best practice. Many of Australia's peers have similarly grappled with the challenges presented by CAR T-cell therapies, which straddle traditional regulatory pathways and create new challenges for reimbursement decisions (discussed above). Most countries have ultimately responded with comprehensive reviews of regulatory settings and the creation of specific regulatory pathways for cellular and genetic therapies (Figure 5.5). The UK has similarly identified funding sources to improve timeliness and equity of access and a process for site accreditation.

Figure 5.5: Processes and funding from international peers



Ideally, access to CAR T-cell therapies in Australia, which is currently concentrated in the East, should be extended nationally over time as part of the development of a National Comprehensive Cancer Centre Network as contemplated by the draft ACP. It is noted, of course, that as a highly specialised service there will be minimum case volume considerations that will inform the commissioning of services to ensure quality and safety.

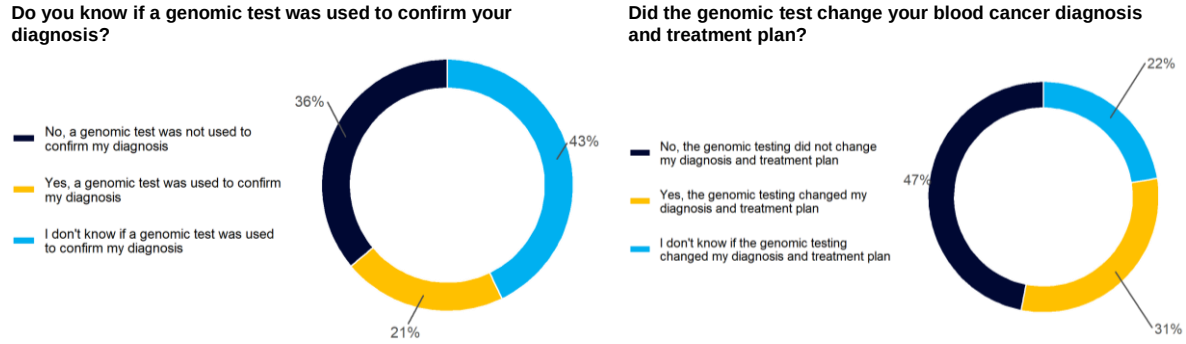
As part of a wider evidence harmonisation strategy, the CPMRD should work with the Leukaemia Foundation, the Blood Cancer Taskforce and state and territory governments to:

- Horizon scan for clinically significant cellular and genetic therapies, liaising with the Blood Cancer Taskforce for clinical expertise and recommendations as needed
- Work with the lead national body for clinical guidelines (e.g., either eviQ or Cancer Council, which would liaise with the Blood Cancer Taskforce for clinical expertise) to develop and maintain Australian clinical guidelines for blood cancers
- Coordinate the commissioning of CAR T-cell therapy services across Australia's National Comprehensive Cancer Centre Network, including considerations for manufacturing quality and safety, workforce development, patient travel costs and funding in accordance with the National Long Term Health Reform Agreement.

Genomic testing as the standard of care

While access to subsidised tests has improved since the development of the first SoTN and the NAP, many novel diagnostics remain unfunded and inconsistently used, even though they are the standard of care across blood cancer. The main gaps include FISH tests and molecular tests to identify biomarkers of clinical significance (Table 5.1).

Figure 5.6: Patient perspectives on genomic testing



Source: People Living with Blood Cancer Survey 2022. See Appendix B.

Similar to the first People Living with Blood Cancer survey, the use of genomic tests to diagnose a blood cancer was reported to be inconsistent, even though many patients were aware that the test had changed their diagnosis and treatment plan (Figure 5.6).

Developing the required evidence base to support a Medicare Benefits Schedule (MBS) item number can be time-consuming, complex and expensive. Compounding upon difficulties, responsibility for preparing MSAC applications often falls to busy specialists. Applications are also hindered by a lack of agreed diagnostic guidelines for the Australian context, which could be used to support an application to MSAC. But again, without clear governance for the development of guidelines this has fallen to the NGO sector, which lacks the funding and, absent a unified approach, authority to develop them.

As explored in the NAP, major challenges also exist in the delivery of diagnostic services for blood cancers that are defined as minimum standard of care by blood cancer clinical guidelines.[75] Revision rates in genomic testing can be high, particularly at non-academic treatment centres,[76] and timeliness remains a significant concern for patients and clinicians. For example, the median reporting time for fluorescence in situ hybridisation (FISH) testing has been reported to be nine calendar days, but the range for reporting time for cancer patients was reported to be anywhere from one to 56 calendar days.[77] Stakeholders indicated that this may be a conservative estimate, with FISH testing for myeloma reported to routinely require 'up to six months'.

For blood cancer patients, these diagnostics are the standard of care and should be funded through the MBS.

Importantly, the Zimmerman Report echoed this conclusion; it called for the Federal Government jointly fund a nationwide genomics testing program with the states and territories, run through the CPMRD.

As part of a wider evidence harmonisation strategy, the CPMRD should work with the Leukaemia Foundation and Blood Cancer Taskforce to:

- Horizon scan for clinically significant novel diagnostics
- Work with the lead national body for clinical guidelines (e.g., either eviQ or Cancer Council, which would liaise with the Blood Cancer Taskforce on technical input) to develop and maintain Australian diagnostic guidelines for blood cancers
- Coordinate evidence development for clinically important diagnostics with industry and clinicians
- Support a nationwide testing program (per the Zimmerman Report) or MBS funding for diagnostics to ensure timely and equitable access to standard of care tests
- Work with Cancer Australia in the development of a national cancer data ecosystem and cancer data performance framework to promote adherence to clinical best practice in blood cancer diagnostics.

Continued limitations in Australian donation of bone marrow

[75] See Chapter 3.
[76] While data on Australian revision rates are not available, National Action Plan working group members indicated challenges in Australian clinical practice mirrored overseas evidence; see for example: Bowen, J.M., Perry, A.M., Laurini, J.A., Smith, L.M., et al, 2014, Lymphoma diagnosis at an academic centre: rate of revision and impact on patient care, British Journal of Haematology, 166, 202-208, doi: 10.1111/bjh.12880.
[77] FISH refers to a cytogenetic technique which enables targeting of specific chromosomal locations within the nucleus of a cell. RCPA, 2018, *Health Genomics Survey 2017*, Final Report, p 55.

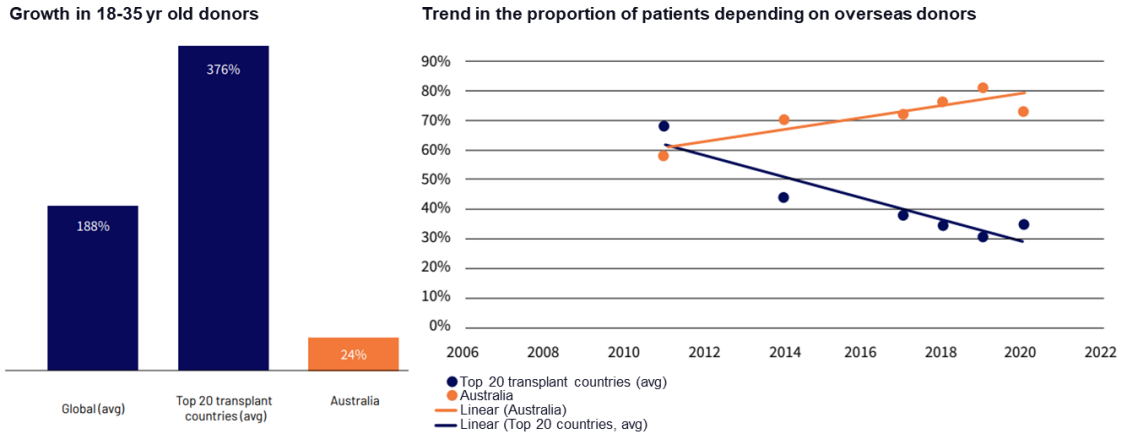
In addition to the wider complexities in the regulatory and technology landscape, a further challenge relates to accessing stem cell transplants.[78]

For many patients, a stem cell transplant is a last resort therapy which, if successful, provides a long term cure for their blood cancer. A patient must be matched with an appropriate Haemopoietic Progenitor Cell (HPC) donor, just as a blood transfusion can only be received from a person with a matching blood type. Patients can be matched with family members (matched related donors) or people from outside their families (matched unrelated donors). If no family members are suitable donors for a patient, then patients will receive National HPC transplant from a matched unrelated donor. The Australian Bone Marrow Donor Registry (AMBDR) maintains a registry of potential matched unrelated donors.

Critically, the list of potential donors registered with the ABMDR has been reducing over time and lacks sufficient demographic diversity to meet the needs of Australia's increasingly culturally diverse population. The Australian donor pool was reported by the ABMDR to be predominantly Caucasian, middle-aged and skewed towards female donors, with limited representation of males (particularly young males) and many ethnic groups

As a result, over 80 per cent of Australian unrelated transplants depend on the ABMDR securing a supply of HPC from an overseas registry (Figure 5.7), such as the ZKRD – the German National Bone Marrow Donor Registry. The dependence on international registries has been increasing at a rate of five per cent per year according to the ABMDR, and is substantially more expensive than domestic donors due to the significant challenges associated with the quality and safe transport of HPC internationally. Reflecting risks associated with importing donations made overseas, fewer Australian transplants involving unrelated donors occurred during the pandemic period (2020-21).[79]

Figure 5.7: Australia's increasing dependance on international donors, in contrast to international peers



Source: ABMDR, 2022, ABMDR Annual Report 2021-2022.

In 2021, federal and state governments recognised the need for Australia to become more donor self-sufficient in their National HPC Framework report; correspondingly, the ABMDR has estimated that 100,000 new HPC donors must be recruited within the next five years.

To achieve this target, the *Strength to Give* program has attempted to promote greater enrolment; however, support has varied across time. For example, as one NGO stakeholder noted, there remains limited awareness of the need for bone marrow donors:

[78] Stem cell transplants, which are called stem cell or haemopoietic progenitor cell (HPC) transplants involve a patient receiving a transfusion of either their own stem cells, which is called an autologous stem cell transplant, or stem cells from a compatible or 'matched' donor, which is called an allogenic stem cell transplant.
[79] ABMDR, 2022, ABMDR Annual Report 2021-2022.

> *The need to sign up to be a bone marrow donor has slipped out of the public's consciousness a little. This is quite topical - someone in my community is in need, they're searching and doing social media posts in hope to find a match. You have people who don't have a match and are awaiting treatment. The psychological impact can be huge. The other question is - are the current criteria relevant? I think its focused on people aged 18-35 years old.*
>
> - *NGO stakeholder*

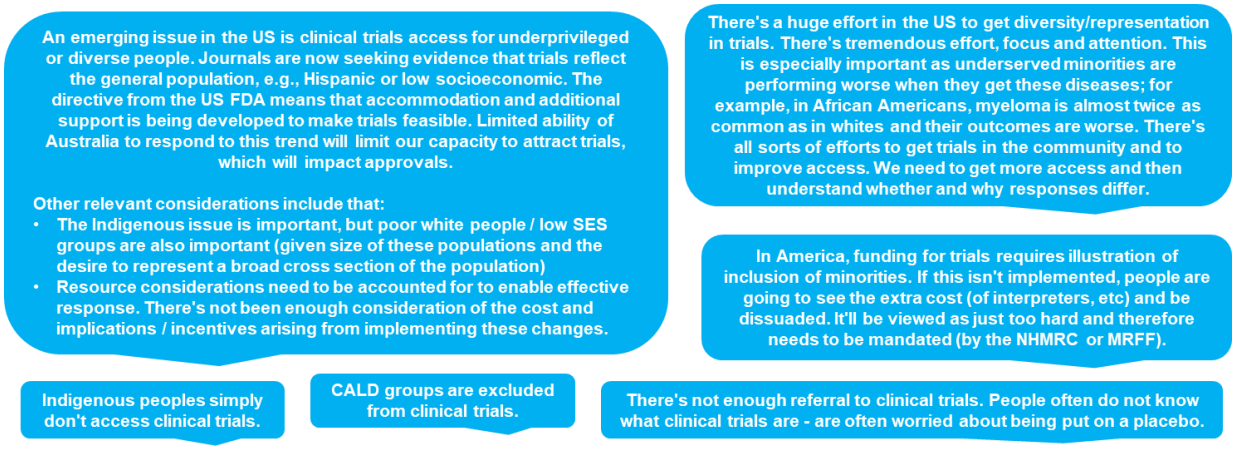
New, proactive strategies are needed for the recruitment, education and retention of ideal donors (young, male and ethnically diverse) to meet contemporary clinical trends and growing demand, so that the needs of Australian patients are met and Australia's reliance on internationally sourced donors is reduced.

Improve awareness of and access to clinical trials

While government has improved funding for regional clinical trials infrastructure and access to tele-trials through COVID-19, there remains significant scope to improve the number, diversity, and equitable trial participation.

As noted by the NAP, many intersecting factors can influence the availability and uptake of clinical trials, including: poor understanding of the benefits of trials; patient travel costs; limited clinician time; limited availability of trials due to funding and capacity barriers for investigator-led trials and market failures for industry-led trials; governance and ethical inefficiencies; and eligibility criteria barriers.[80] These barriers tend to compound for disadvantaged groups, including regional and remote patients, geriatric patients, people from culturally and linguistically diverse backgrounds and people from low socioeconomic status backgrounds.

Figure 5.8: Stakeholder perspectives on inclusion in clinical trials



Source: Insight Economics consultation with stakeholders, conducted in late 2022.

The 2022 Survey of People Living with Blood Cancer found that, similar to the 2018 study, only 21 per cent participated in a clinical trial, and that among people who did not participate in a clinical trial the primary reason was that it was not discussed (74% of respondents), while a further 10 per cent indicated that their specialist had looked for a trial but no relevant clinical trial was available (Figure 5.9).

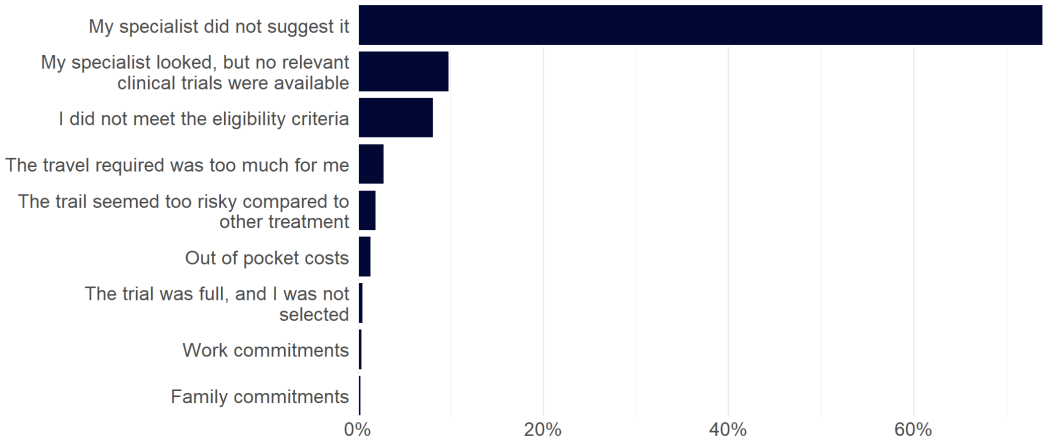
This is consistent with international studies. For example, a 2016 ASCO report noted that less than five per cent of adult patients with cancer enrol in cancer clinical trials even as studies also suggest more than 70 per cent of Americans are estimated to be inclined or very

[80] ALLG and HSANZ submission to the National Action Plan for Blood Cancer.

willing to participate in clinical trials.[81] Similarly, the Journal of the National Cancer Institute in 2017 noted:

> *The low rates of participation in cancer trials (<5 per cent), especially for minorities, the elderly, low-income individuals, and those living in rural areas, are clear indications of the barriers posed by segmentation of clinical research to geographically dispersed sites.*
>
> *Indeed, the recent right-to-try debates suggest that barriers to gaining convenient access to experimental therapies, rather than patient preferences, are the prohibitive force behind low participation in cancer trials.*[82]

Figure 5.9: What was the reason you did not participate in a clinical trial?



Source: People Living with Blood Cancer Survey 2022. See Appendix B.

Despite demonstrated willingness to participate in clinical trials, members of the blood cancer community reported that patient recruitment is incredibly challenging under current arrangements, with many trials closing due to an inability to recruit and retain patients. For example:[83]

- A 2014 survey conducted by Clinical Trials Connect found that only 20 per cent of trials met their recruitment deadline
- Medicines Australia indicated that more than 50 per cent of sites did not meet their recruitment requirements, pointing to fragmentation of the sector and the need for better coordination of effort at the national, state and territory level
- A review of clinical trial performance for the Commonwealth Department of Health by Ernst and Young identified a lack of streamlined infrastructure as a key barrier to recruitment and retention in Australian clinical trials. Trial site staff reported that finding participants that fit complex eligibility criteria is a major barrier to recruitment.

The development of a performance framework through the national cancer data ecosystem represents an important tool to promote a 'research ready' culture in clinical practice and ensure that patients are consistently made aware of clinical trial options. Alongside the

[81] Unger, J.M., Cook, E., Tai, E., et al., 2016, The Role of Clinical Trial Participation in Cancer Research: Barriers, Evidence, and Strategies, ASCO Educational Book, accessed at: https://ascopubs.org/doi/full/10.1200/EDBK_156686.
[82] Khozin, S., Blumenthal, G.M., Pazdur, R., 2017, Real-world Data for Clinical Evidence Generation in Oncology, J Natl Cancer Inst, 109(11), doi: 10.1093/jnci/djx187. Oncology Center of Excellence, Food and Drug Administration, Silver Spring, MD.
[83] As referenced in: Zimmerman Report, 9. Clinical Trials, para 9.104; Medicines Australia, 2020, Submission to the Inquiry into approval processes for new drugs and novel medical technologies in Australia; EY, 2016, Scoping and analysis of issues in recruitment and retention in Australian clinical trials; You, K.H., Ahern, E., Wyld, D., et al., 2022, Scoping to analyze oncology trial participation in Australia, Seminars in Oncology, 49(2).

development of KPIs for clinical trials, consumer navigation services should be designed to improve patient awareness of trials and support reviews of patient eligibility. The Leukaemia Foundation and Cancer Australia should work together to improve the quality of consumer navigation services to better support patient participation in clinical trials, particularly in underserved groups.

5.3 Next horizon priorities

The Survey of People Living with Blood Cancer indicated that enabling access to novel treatments was ranked as a lower order strategic objective relative to achieving best practice in the health system and funding blood cancer research.

While a number of reform initiatives have been set into motion, there is significant work to be done to improve the timely and equitable access to novel and specialised therapies.

Table 5.2: Summary of actions, progress to date and implementation considerations

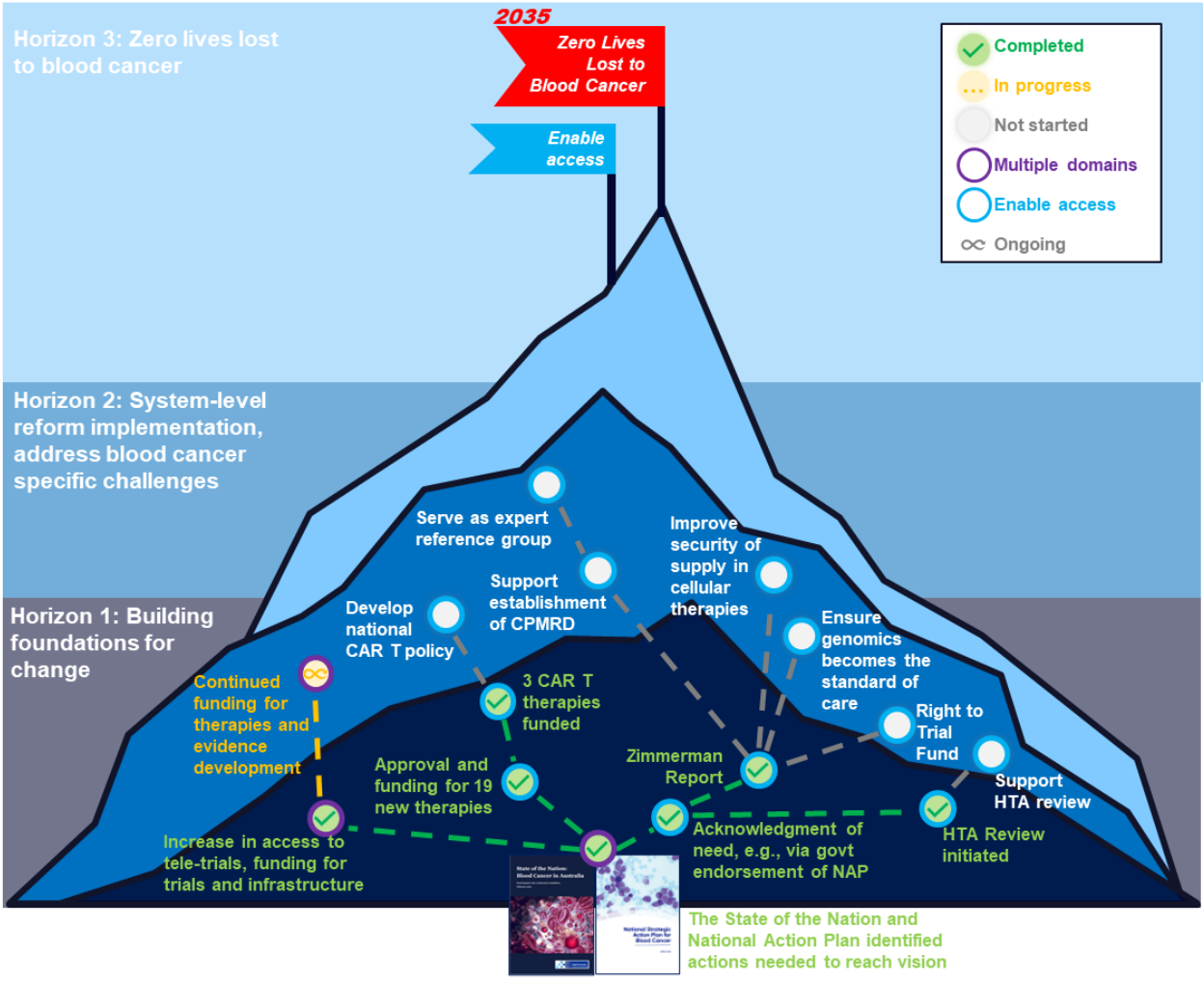
Summary of actions	Progress to date	Implementation considerations
Expand access to clinical trials, including for regional and remote patients, through key performance indicators for discussion of clinical trial options with patients	✓ Commitment within Australian Cancer Plan to performance management framework ✓ National Action Plan endorsement of recommendation (Actions 4.1 and 4.2) ✓ The National One Stop Shop and National Clinical Trials Front Door ✓ 2019 Rural, Regional and Remote Clinical Trial Enabling Infrastructure grant opportunity ✓ Increased use due through COVID-19	With benefits to all cancers from a systems level approach and commitment by governments to lead development of performance framework and implement clinical trials reforms, the lead partner should be the Australian Government, with the Leukaemia Foundation and National Blood Cancer Taskforce supporting in a partnership role.
Make genomics the standard of care	✓ National Action Plan endorsement of recommendation (Action 2.3) ✓ MSAC bid for MBS item number	Access agenda needs to explicitly tackle: • Right to Trial program • National CAR-T policy • Security of supply of cellular therapies • Genomics as the standard of care Reflecting the intensity of research and technology change in blood cancer therapies, which is leading wider cancer field, particularly in cellular therapies, the Blood Cancer Taskforce is well-placed to serve as an expert advisory group to an independent CPMRD.
Expand access to novel and specialised therapies through Right to Trial program	✓ Enabling Access Working Group established in National Blood Cancer Taskforce with work program identified to improve equitable access to novel and specialised therapies (Action 4.3) ✓ Zimmerman Report recommendations support for Right to Trial program and HTA reform	

As a therapeutic area that is leading the field in the development of novel diagnostics and treatments, the Leukaemia Foundation and Blood Cancer Taskforce have a strong **leadership role** to play in partnership with Australian Governments to provide the patient and clinical expertise needed to address the barriers that have now been widely and repeatedly acknowledged by government.

Having established this expert body and evidence base through the NAP, the Leukaemia Foundation, working in partnership with the Blood Cancer Taskforce, should work with government to:

- Support the establishment of an independent CPMRD
- Support the HTA review in its development of updated guidance on levels of evidence, additional regulatory and reimbursement pathways, and a revised approach to funding using coverage with evidence development (or managed entry access)
- Serve as an expert reference group on clinically important diagnostics and therapies in blood cancer as part of the horizon scanning to be undertaken by the CPMRD
- Support the establishment of a Right to Trial Fund, as recommended by the Zimmerman Report, with blood cancers serving as a pilot for the development of the fund over the next two years
- Support the development of a national approach to CAR T-cell services
- Support the development of genomic testing as the standard of care in blood cancer.

Figure 5.10: Priorities in enabling access and progress to date



Note: Provides a summary of progress to date and priorities going forward. The following acronyms are used: Australian Cancer Plan (ACP), Centre for Precision Medicine and Rare Diseases (CPMRD), Health Technologies Assessment (HTA), Chimeric Antigen Receptor T-cell therapy (CAR T), National Action Plan (NAP). Source: Insight Economics.

Chapter 6

Priorities for Action

The State of the Nation set a goal that by 2035 there would be zero lives lost to blood cancer. The aim being to remove barriers to access and addressing inequality in survival outcomes. This may be achieved by increasing access to information, best practice treatment and supportive care, to maximise quality of life.

These long-term ambitions necessitate significant systems reform and sustained effort to drive execution through partnerships with federal and state governments and the blood cancer community.

The Leukaemia Foundation, in partnership with the blood cancer community have worked to develop critical capabilities needed to catalyse systems reform. Still, there remains important work to be done to realise the ultimate vision for the strategy.

This chapter summarises the key priorities for action over the next implementation horizon of the zero by 2035 strategy and partnerships for change.

6.1 The 2035 vision remains the same

Founded in 1975 with a vision to cure blood cancer and a mission to care for people living with blood cancer, the Leukaemia Foundation continues to support countless people living with blood cancer and their loved ones.

This update of the first SoTN continues the Leukaemia Foundation's commitment to improve outcomes and quality of life for people impacted by blood cancer, and reaffirms the Leukaemia Foundation's long-term ambition for:

Zero lives lost to blood cancer by 2035,
underpinned by zero preventable deaths,
regardless of geography or background,
through equitable access
to best practice treatment and care
for all Australians.

6.2 Key priorities for implementation

To achieve this vision, the Leukaemia Foundation proposed four strategic objectives and a plan to work in partnership with government and the blood cancer community to coordinate effort that will enable the achievement of this goal.

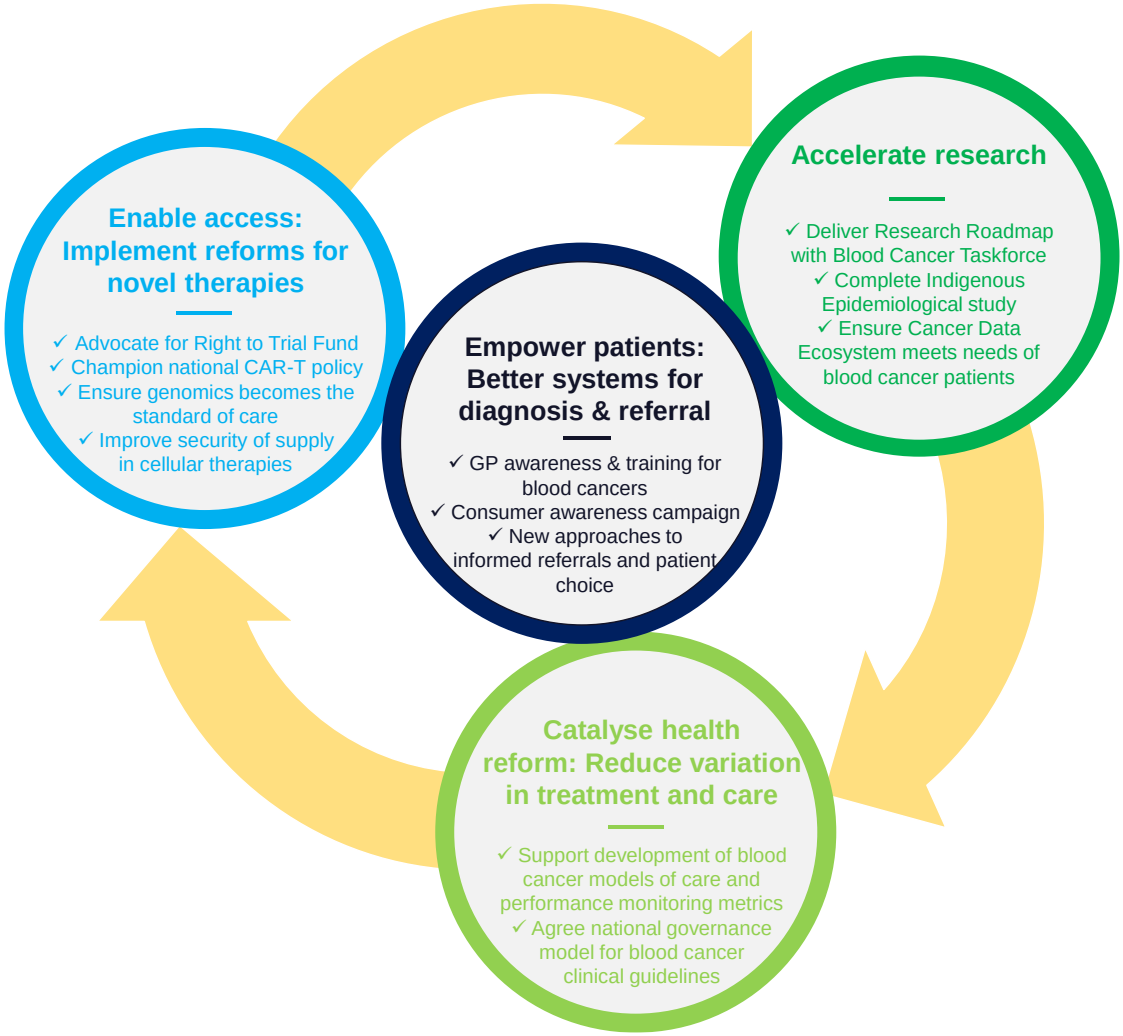
To date, the Leukaemia Foundation has brought the blood cancer community together through the first ever Blood Cancer Taskforce and NAP. The Leukaemia Foundation has also worked closely with the Federal Government to build on ideas to improve outcomes for all people with cancer through the ACP. This includes initiating agendas to improve access to novel and specialised therapies.

The Leukaemia Foundation will work in a partnership role with government where it is leading health system reforms to ensure these reforms meet the needs of blood cancer

patients. It will work in a leadership role in areas that are 'blood cancer specific' or where blood cancer leaders are best placed to champion action, in collaboration with the Blood Cancer Taskforce, the Cancer Council, Myeloma Australia, Lymphoma Australia and other partner organisations.

Examples of actions where the Leukaemia Foundation can adopt a partnership role in the execution of system-level reforms include reform initiatives being led by Cancer Australia in the ACP. Key 'blood cancer-specific' priorities for action over the next horizon are identified below.

Figure 6.1: Key priorities for implementation over the next horizon



Strategic Objective 1: Empower Patients: Better systems for diagnosis & referral

A number of key actions identified by the first SoTN and NAP have been taken up by the ACP. It is proposed that the Leukaemia Foundation take a **partnership role** to ensure implementation of the ACP meets the needs of blood cancer patients against the following key areas of action:

- *Consumer navigation and cancer information service* – Actively engage in the co-design of the service and ensure patients are appropriately triaged to blood cancer support services from diagnosis to get appropriate support throughout their journey

- *National cancer data ecosystem and performance framework* – Actively engage with Cancer Australia in the design of cancer data frameworks, which would integrate clinical, population and Patient Reported Outcome data, and ensure performance metrics of quality and safety meet the needs of people living with blood cancer (e.g., written care plans, referrals to supportive care)

Areas of need that have been identified by the first SoTN and NAP that are not going to be addressed through other reform efforts include where the Leukaemia Foundation must take a **leadership role**:

- *Education and training of blood cancer signs and symptoms for GPs* – Work with Cancer Australia to develop disease-specific content that supports improved understanding of blood cancers
- *Systems for informed consent and patient choice in referrals* – Work with the blood cancer community to develop an informed consent and patient choice directory of blood cancer services
- *Consumer awareness of blood cancer symptoms* – Implement a campaign to improve community awareness of blood cancers and their rights as a patient.

Strategic Objective 2: Catalyse health services reform to achieve best practice

The Leukaemia Foundation can take a **partnership role** to ensure relevant ACP initiatives meet the needs of blood cancer patients. It should also work in partnership with the Cancer Council and wider NGO community to tackle reforms to prevent financial hardship, a key priority of the Cancer Council.

Areas of need that have been identified by the first SoTN and NAP that are not going to be addressed through other reform efforts, however, where the Leukaemia Foundation must take a **leadership role**, include:

- *Support the development of blood cancer models of care and clinical guidelines for blood cancer* – Build on Cancer Australia's work to develop innovative models of care within the National Comprehensive Cancer Network and develop additional clinical guidelines for blood cancer in partnership with the Blood Cancer Taskforce
- *Champion a national approach to clinical guidelines governance and funding* – Working with the Blood Cancer Taskforce, and building on Phase 1 funding for the development of a blood cancer clinical guideline, the Leukaemia Foundation should advocate for an efficient and sustainable, national approach to clinical guidelines development.

Strategic Objective 3: Accelerate research

The Leukaemia Foundation should work in a **partnership role** to ensure the national cancer data ecosystem is designed and developed in such a way that researchers are easily able to access natural history, population, clinical and patient reported data to enable the acceleration of high impact blood cancer research and international research collaborations.

In the short term, the Leukaemia Foundation will take a **leadership role** in:

- Working with the Blood Cancer Taskforce to deliver the Research Roadmap
- Delivering the epidemiological study of blood cancer in Aboriginal and Torres Strait Islander communities

- Ensuring the National Cancer Data ecosystem is developed to meet the needs of blood cancer patients, clinicians and researchers.

Over the longer term, the Leukaemia Foundation, Blood Cancer Taskforce, blood cancer community and state and federal governments including NHMRC and MRFF, should partner to fund the implementation of the Research Roadmap.

Strategic Objective 4: Enable access to novel and specialised therapies

While a number of reform initiatives have been set into motion, there is significant work to be done to improve the timely and equitable access to novel and specialised therapies.

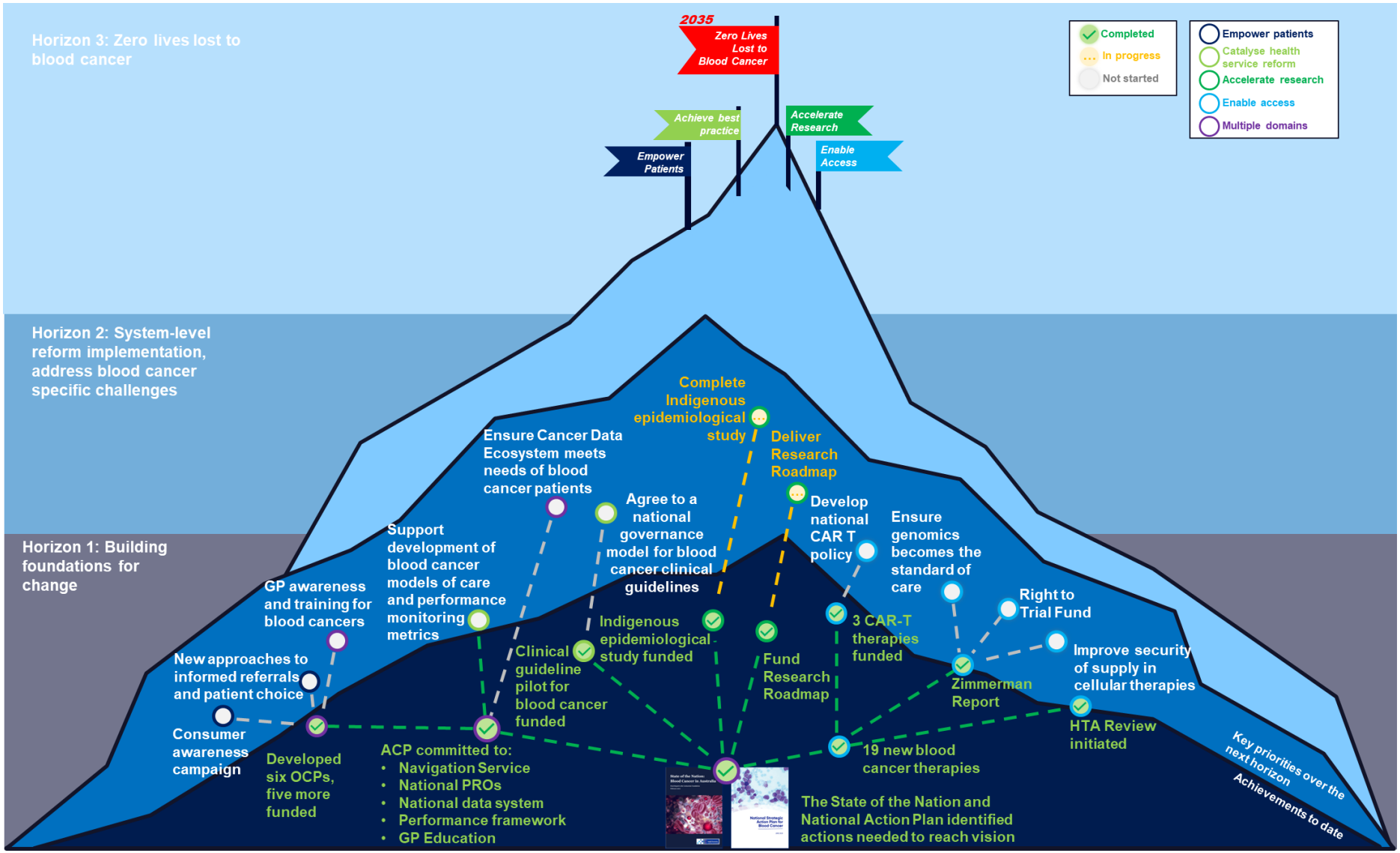
As a therapeutic area that is leading the field in the development of novel diagnostics and treatments, the Leukaemia Foundation and Blood Cancer Taskforce have a strong **leadership role** to play in partnership with federal and state governments to provide the patient and clinical expertise needed to address widely acknowledged barriers. The Leukaemia Foundation, working in partnership with the Blood Cancer Taskforce, should proactively work with government to:

- Support the establishment of a Right to Trial Fund, as recommended by the Zimmerman Report, with blood cancers serving as a pilot for the development of the fund over the next two years
- Support the development of a national approach to CAR T-cell services
- Support the development of genomic testing as the standard of care in blood cancer.
- Improve the security of supply of cellular therapies.

Conclusion

In focusing on these key areas of action alongside wider health system reforms and working in partnership with the Blood Cancer Taskforce, the Leukaemia Foundation will continue to improve outcomes for people living with blood cancer on the journey to zero lives lost by 2035.

Figure 6.2: Summary of actions to achieve vision



Appendix A
Modelling method and data

A.1 Method overview

The Blood Cancer Projections to 2035 were updated following a bottom-up cohort-component method.

ABS Population Projections for Series A and B were aggregated into 5-year age groups to match AIWH Australian Cancer Database, State Cancer Registry data and PHN data. Prevalence was based on public and custom requested data from Victoria and Queensland Cancer Registries.

Rates of incidence and incidence growth rates based on AIHW data by sex and blood cancer sub-type were applied to ABS projections by five-year age-group from 2018 to 2035 and then stratified into PHN using ABS data.

Mortality was projected based on the application of age-based survival curves at 1-year, 5-year and long run conditional survival age-based survival data was obtained from the State Cancer Registries through a customised data request to the incidence and prevalence cohorts. Using long run conditional survival data from the AIHW only a proportion of the deaths of long run survivors were attributed to blood cancer.

The model was built to allow for sensitivity analysis in:

- Higher Population Growth (Series A Projections, as opposed to Series B which are reported)
- Under incidence in reporting to State Cancer registries (the report assumes no under-reporting occurs)
- Alternative incidence growth rates (the report presents five-year average growth from 2009-2014 (latest data) and zero growth, with 10-year or 20-year averages producing astoundingly high expectations for the incidence and prevalence of blood cancer)
- Variation in incidence by PHN
- Variation in survival outcomes by State, by age group by sex by blood cancer sub-type.

A.2 Key outputs

The results from the Blood Cancer Projection Model include:

- Incidence by cancer sub-type, age and sex by PHN, State and nationally for the years 2022-2035
- Prevalence by cancer sub-type, age and sex by PHN, State and nationally for the years 2022-2035
- Mortality by cancer sub-type, age and sex by PHN, State and nationally for the years 2022-2035

- YLL by cancer sub-type, age and sex by PHN, State and nationally for the years 2022-2035
- YLD by cancer sub-type, age and sex by PHN, State and nationally for the years 2022-2035.

A.3 Key data

Variable	Data source
Population projections	ABS Cat. No. 3222
Incidence	AIHW Australian Cancer Database, including Australian Cancer Incidence and Mortality Books for each sub-type ABS Cancer Incidence and Mortality by PHN
Prevalence	Victorian Cancer Registry Queensland OASys
Survival and mortality	Victorian Cancer Registry, Cancer Institute NSW, Queensland OASys, Tasmanian Cancer Registry, AIHW Cancer in Australia 2017. Data for WA and SA were not able to be made available in the time to write the report.

A.4 Best practice scenario modelling

Incidence, prevalence and mortality modelling has been developed based on the aggregation of sub-type models built for each gender; that is, there are separate model projections for:

- AML Males
- AML Females
- ALL Males
- ALL Females
- CML Males
- CML Females
- CLL Males
- CLL Females
- NHL Males
- NHL Females
- Hodgkin Males
- Hodgkin Females
- Myeloma Males
- Myeloma Females
- MDS Males
- MDS Females.

Within each of these models, survival curves have been developed for the following age cohorts: (0-14), (15-44), (45-54), (55-64), (65-74) and (75+) for each jurisdiction based on the latest available average five-year data from Australian cancer registries and the AIHW.

The best outcome by jurisdiction for each age cohort was identified and then applied nationally. For example, if one jurisdiction consistently achieved 5-year survival of 50 per cent compared to a national average of 45 per cent (hypothetical) then the 50 per cent outcome was applied to this cohort. When variation in outcomes are eliminated for each cohort (stratified by age, gender, blood cancer sub-type and region) based on latest available cancer registry data, this shows that up to 29 per cent of projected blood cancer mortality could be prevented.

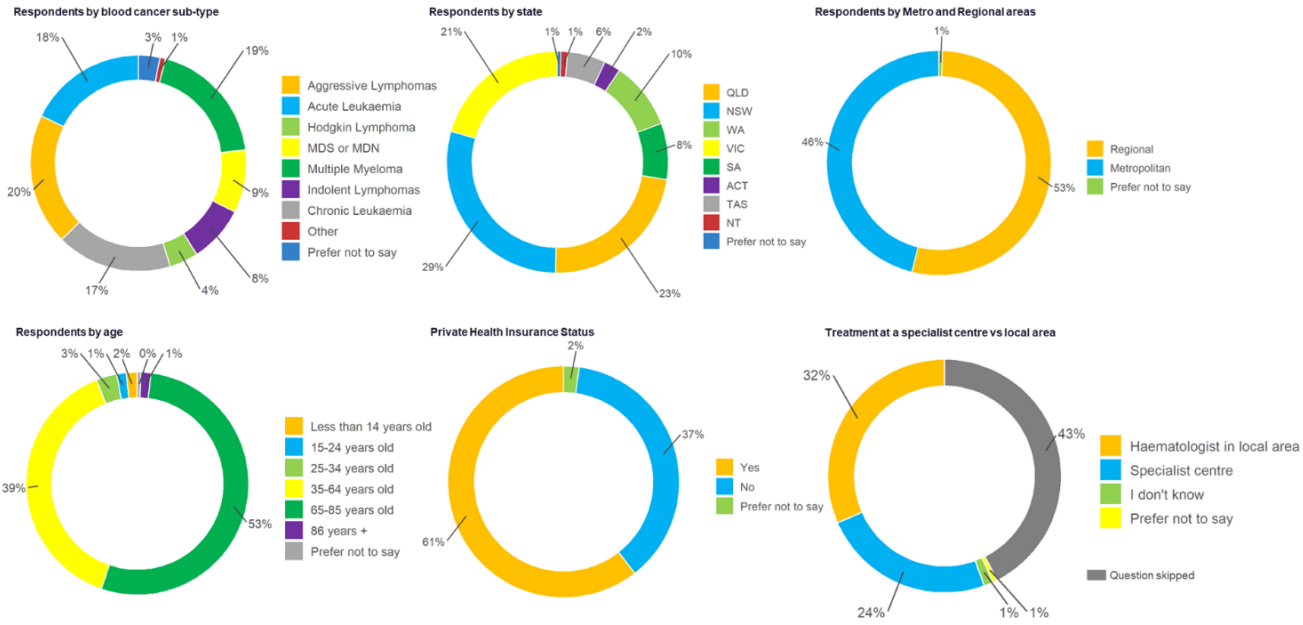
Appendix B
Survey of People Living with Blood Cancer

B.1 Overview

There were 4,600 respondents to the 2022 survey, as compared to 3,200 in 2018.[84] The sample is statistically significant, with 95 per cent confidence the true value would be within +/- 1.29 per cent margin of error.

The survey delivered a good representation of sub-types, States, regional status, age and private health insurance status.

Figure B.1: Summary of respondents



Survey logic was used to vary which questions patients received. The survey logic was dependent on the question "Have you been newly diagnosed or experienced a relapse or transformation with a blood cancer since 2018?" (2018 and later). Respondents who were diagnosed prior to 2018 (pre-2018) received fewer questions.

Table B.1: Non demographic question list

Note	Questions
2018 and later	What was the approximate length of time between when you first presented to your GP with symptoms and your first appointment with a haematologist?
2018 and later	Were you referred to other specialists before your referral to the haematologist?
2018 and later	Do you know if a genomic test was used to confirm your diagnosis? (e.g testing the genes of your specific cancer (this is not the same as genetic testing for cancer risk)

[84] Defined as separate survey respondents, i.e., a respondent who answers at least one demographic question.

Note	Questions
2018 and later	Did the genomic test change your blood cancer diagnosis and treatment plan?
2018 and later	Was a bone marrow biopsy used to confirm your diagnosis?
2018 and later	At the time when you were diagnosed did you have a good understanding of your diagnosis?
2018 and later	Did you have a good understanding of your treatment plan?
2018 and later	Did you know that Optimal Care Pathways (OCP) for some blood cancers are now available?
2018 and later	What lifestyle challenges have you experienced as a result of your blood cancer? (select all that apply)
2018 and later	Did your treatment plan include a discussion of supportive care in additional to clinical care (Select all that apply)
2018 and later	Did you know where to go if you had questions?
2018 and later	Where did you go if you needed more information? (Select all that apply)
2018 and later	What kind of information do you wish you had received during your treatment planning? (Select any that apply)
2018 and later	What types of treatment did you have? (Select all that apply)
2018 and later	Was your therapy:
2018 and later	Were you able to access transport and accommodation support if needed?
2018 and later	Was there good coordination of care between your specialist and your GP?
2018 and later	Have you received any active treatment in the last 18 months?
2018 and later	Did you receive information or help during active treatment regarding: (Select all that apply)
2018 and later	In hindsight, would more help in any areas have helped you during active treatment? (Select any that apply)
2018 and later	After being diagnosed, did you receive information or help regarding: (Select all that apply)
2018 and later	In hindsight, would more help in any areas listed below have helped you? (Select any that apply)
2018 and later	Did you face any significant out of pocket costs during your diagnosis and/or treatment?(Out of pocket costs are costs that you personally fund that are not covered by the hospital or insurance.)
2018 and later	How much did you incur in out of pocket costs in total?
2018 and later	What were the major sources of cost?
2018 and later	How did you cover out of pocket costs, excluding any payments you may have received from government sources? (Select all that apply)
2018 and later	If you would like to provide more detail about the financial impact of your out of pocket costs, please do so here.
2018 and later	Did you access financial assistance from the government (e.g. JobSeeker)
2018 and later	At what point in your cancer diagnosis and treatment did you access financial assistance from the government?
2018 and later	Were you able to continue working through your treatment?
2018 and later	Have you returned to work since your diagnosis?
2018 and later	Was your carer able to continue working through your treatment?
2018 and later	Did you participate in a clinical trial at any point of your treatment?
2018 and later	What aspect of your treatment was the focus of the clinical trial?
2018 and later	Where did you participate in the clinical trial?
2018 and later	What was the reason you did not participate in a clinical trial? (Select all that apply)

Note	Questions
All respondents	Has COVID had an impact on your treatment and care? (Select all that apply)
All respondents	Are you aware that COVID vaccines may be less effective in blood cancer patients that for the wider community?
All respondents	Are you aware there is a treatment called Evusheld that can reduce the risk of severe COVID infection for blood cancer patients?
All respondents	Have you been provided with Evusheld? If so, how often have you received it?
All respondents	What are the highest priority actions to better empower patients and their families?
All respondents	What are the highest priority actions to improve the health care systems for people living with blood cancer?
All respondents	What are the highest priorities to accelerate research and improve access to new therapies?
All respondents	What should be the highest priority area for action for governments? (order from 1 highest priority to 4 lowest priority)
All respondents	Is there anything else you would like us to know?
All respondents	I would like a Leukaemia Foundation Blood Cancer Support Coordinator to contact me
All respondents	Please leave your name and contact details below

Appendix C
Stakeholder consultation

C.1 List of stakeholders consulted

Over the period spanning October-December 2022, Insight Economics undertook 24 consultations. As part of the consultation process, stakeholders were invited to share their thoughts and data regarding progress to date and key priorities going forward.

Table C.1: Stakeholders consulted

Focus	Stakeholder, role and organisation
Leukaemia Foundation	Chris Tanti, CEO Lucio Di Giallonardo, Chair of Board Mark Cormack, Board Member
Taskforce leadership	Prof John Seymour, Peter Mac Prof David Joske, Solaris, COSA Prof Maher Ghandi, UQ, TRI Delaine Smith, ALLG Prof Steven Lane, QIMR Prof Andrew Roberts, WEHI
Clinician perspectives, CAR T-cell therapies and guidelines	A/Prof Michael Dickinson, Peter Mac
Clinician perspectives, care standards and private service provision	Dr John Bashford, Icon Group
Cancer Australia	Dr Anna Boltong Cindy Toms
Dept of Health	Paul Salmond Melissa Shearing Jonathan Ratcliffe
Vic Health	Kathryn Whitfield
CAR T-cell therapies	Prof Joy Ho, Royal Prince Alfred Hospital
Data	Prof Brendon Kearney, Royal Adelaide hospital
Research partnerships	Lee Greenberger, LLS
Blood cancer in CALD communities	Daniel Coase, FECCA
Blood cancer in Aboriginal and Torres Strait Islander communities	Prof Gail Garvey, UQ Dr Kate Armstrong, NACCHO Rebecca Reese, NACCHO
Exercise oncology	Jane Turner, Sydney Survivorship Centre
Blood Cancer NGOs	Sharon (Millman) Winton, Lymphoma Australia Hayley Beer, Myeloma Australia Kirstee Macbeth, Snowdome
Cancer NGO perspectives	Tanya Buchanan, Cancer Council

Focus	Stakeholder, role and organisation
Consumers	Scott Steinkrug David Hill David Young Peter Freese Tony Veitch Briony Benjamin Sarah Stevens Barry Du Bois
Politician	Dr Mike Freelander

C.2 Consultation brief

State of the Nation in Blood Cancer: 2022 Update

Consultation Brief

Project Background

In 2019, the Leukaemia Foundation launched the *State of the Nation in Blood Cancer*. This important work brought together consumers and Australia's most respected leaders in blood cancer to speak with a united voice for change.

The State of the Nation report set a goal that by 2035 there would be zero lives lost to blood cancer. This would mean, by 2035, zero preventable deaths, through the removal of barriers to access and addressing inequality in survival outcomes. It will also mean, by 2035, zero people living with blood cancer without access to information and best practice treatment and supportive care.

These long-term ambitions necessitate significant systems reform and sustained effort to drive execution through partnerships with Australian governments and the blood cancer community.

By getting blood cancer on the agenda, the Leukaemia Foundation has supported the development of the critical enabling capabilities needed to catalyse systems reform. This has included the establishment of a National Blood Cancer Taskforce and the development of a National Strategic Action Plan for Blood Cancer. Initial progress has been made in the development of six additional Optimal Care Plans (with a further five now funded by government) and federal funding for the development of a national approach to the creation of Australian clinical guidelines for blood cancer. It is also evidence in the listing of 34 medicines on the PBS and $80 million in funding for CAR-T therapy.

Added to this, the Zimmerman report supported *State of the Nation* recommendations for the establishment of program to improve evidence development and access to therapies (Right to Trial Program), as well as wider health technology assessment and regulatory reform, which are now underway.

At the same time, there remains important work to be done to realise the ultimate vision for the strategy. More work is needed to deliver the Phase 1 initiatives identified in the National Action Plan that are fully costed and shovel ready. Work is also needed to ensure significant national reform efforts — like the Australian Cancer Plan and other reform initiatives — are developed to deliver the step-change improvements put forward by the *State of the Nation* report to deliver improved consistency, quality and safety of blood cancer services across Australia.

Thus, three years on — in the context of the COVID-19 pandemic, a recent change of Federal government, and a highly active policy reform environment — it is timely to take stock of progress to date and identify priorities for action over forward horizon.

Questions for discussion

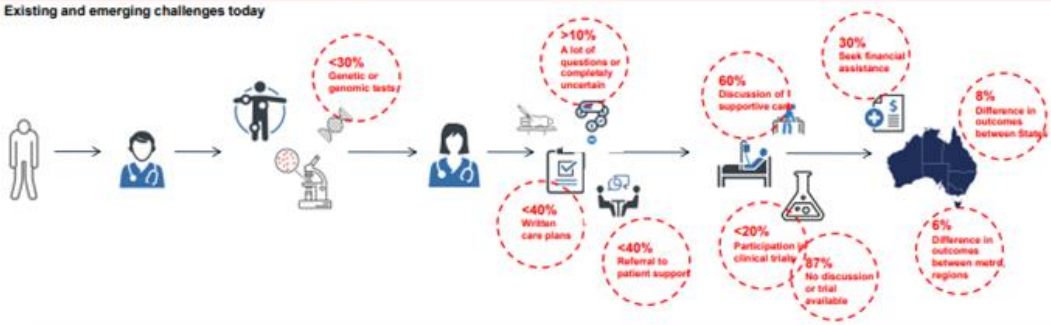
- How has the landscape changed since 2018? **Do the same challenges exist** for patients and carers? How has **COVID impacted on the treatment and care** of Australian patients? Can **learnings** from the response to COVID be applied for better care of patients in the future?
- What are the **implications of national reform efforts**, such as the development of the Australian Cancer Plan and Health Technology Review, for the State of the Nation in Blood Cancer agenda?
- What are the **highest priority areas for action** in the next three years?

1

Insight Economics
+Public Policy +Corporate Strategy

State of the Nation: Key challenges & future vision

Existing and emerging challenges today

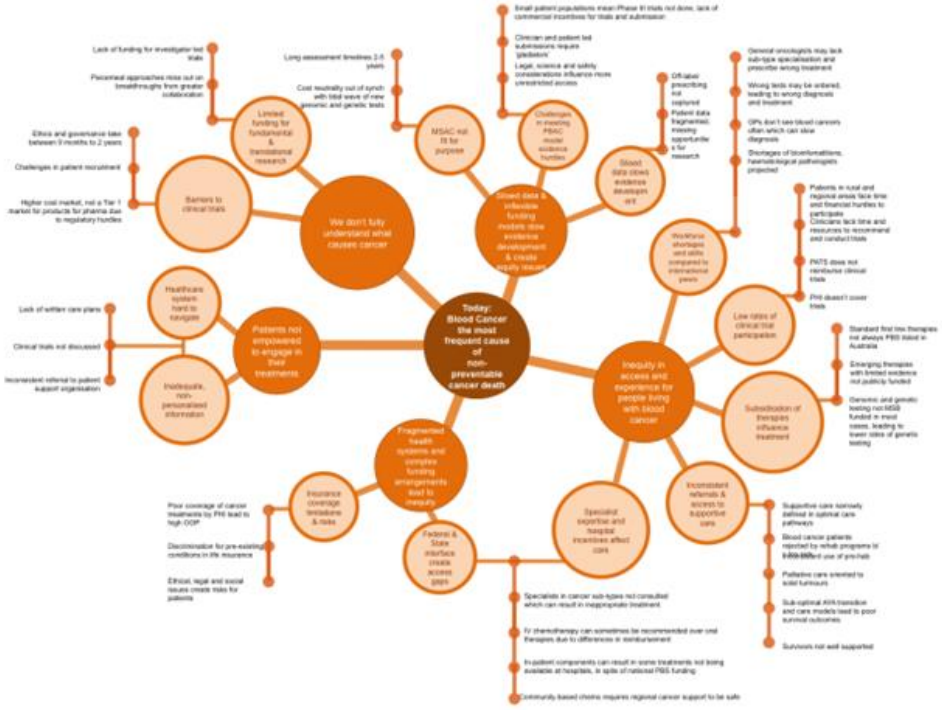


Vision for 2035

2

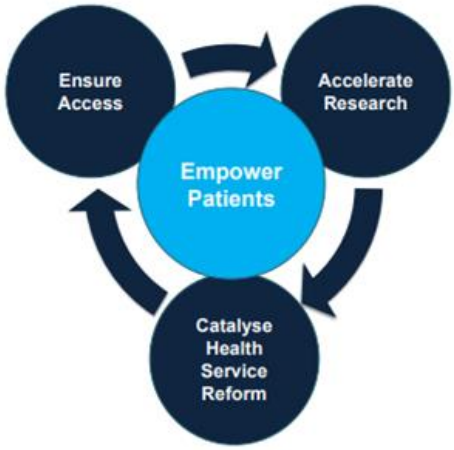
Insight Economics
+Public Policy +Corporate Strategy

State of the Nation: the landscape for blood cancer in 2018



3

State of the Nation: priority areas for action



Actions to Empower Patients

- National information support services delivered to the patient at diagnosis
- Opt-out model for referrals to patient support organisations
- Create a one-stop shop for blood cancers
- Create a complex referral MBS item and referral support tools
- Support the development of Patient Reported Outcomes
- KPIs for written care plans

Actions to Accelerate Research

- Establish an International Blood Cancer Research Mission
- Develop a Real World Evidence Pilot for the MyHealthRecord - Including Patient Reported Outcomes

Actions to Ensure Access

- Make systematic genetic and genomic testing part of the standard of care
- Develop a Right to Trial Pilot Program (Clinical Trials program for rare indications)
- Implement KPIs for clinical trial participation

Actions to Catalyse Health Service Reform

- Address care pathway and clinical guideline gaps
- Develop KPIs for sub-type specialist input to treatment plans
- Develop KPIs for supportive care screening and referrals
- Review of in-patient and out-patient funding arrangements
- Roll-out GP education and decision support tools
- Develop and roll-out a cancer-friendly rehabilitation program
- Support the expansion of community-based care
- Advocate for insurance reform
- Advocate for welfare support, including Centrelink payments reform
- Advocate for patient assisted travel scheme reform

Initial progress made, but work to be done

Little to no progress observed to date

4

Appendix D
Policy context

D.1 Overview of key policy initiatives

Since the first SoTN, several major policy changes have been developed at a federal level. Other major policy changes are also (at the time of drafting) in development. These policy documents are identified in the diagram below.

In focus: State of the Nation (2019)

This State of the Nation: Blood Cancer in Australia report sets out an agenda to help save lives and to ensure that everyone with a blood cancer in Australia has access to the best possible information, treatments and care.

In focus: National Strategic Action Plan for Blood Cancer

The tables below contain the priorities set out in the NAP.

Table D.1: Actions to Empower Patients

Action	Implementation	Timeframe
OBJECTIVE: Empower patients to make informed choices		
1.1 Empower patients to navigate the healthcare system	1.1.1 Develop a patient navigation service to assist people living with blood cancer to navigate the healthcare system. Delivery of this service should be considered as part of the Blood Cancer Information Strategy (Action 1.2). At diagnosis, primary healthcare providers will refer patients to both a haematologist (and/or other specialist) and confirm consent for a referral to a patient navigation service. Consent and referral to a patient navigation service should also be facilitated through specialist services. This will help support patients to know the right questions to ask, connect people with national sources of information about clinical trial opportunities, and connect them to relevant resources to support decision-making or access support. The patient take-up rates of the referral would determine the size of the patient navigation support service required. The service should factor in the unique needs of high-risk groups and patient life stages to best target support.	1-2 years
1.2 Develop a Blood Cancer Information Strategy as part of a broader digital health and information strategy for cancer	1.2.1 Form an inclusive, patient focussed and cross-organisational working group to develop a Blood Cancer Information Strategy, as part of a broader digital health and information strategy for cancer. The aim is to ensure patients can access the right information, at the right time, which is relevant to their diagnosis and personal situation. This information could include: • Information for primary care clinicians, specialists and other healthcare workers • Information to support complex referrals to specialists • Information about supportive care services for patients and carers, including support for financial planning • Connect people to national sources of information about clinical trial opportunities. The working group should include patient organisations, patient advocates, clinicians, researchers and other relevant stakeholders. It will be focussed on the principles and objectives that organisations agree will deliver better outcomes for patients. This action would be supported by a training and change management strategy delivered to primary healthcare workers and specialists.	1 year
1.3 Develop KPIs for written treatment and survivorship care plans	1.3.1 Develop minimum standards or 'template' for written treatment and survivorship care plans, for both acute and chronic blood cancers. Treatment and survivorship plans would be dynamic and remain relevant to the patient at different stages of their diagnosis and treatment and include guidance on supportive care considerations such as managing immunosuppression, community sourced infection, vaccination, diet, exercise, palliative care and any other psycho-social supportive care needs. 1.3.2 The establishment of KPIs would be led by the Australian Commission on Safety and Quality in Health Care and would leverage work already underway by the Commission, in partnership with clinical working groups from each jurisdiction. Once the systems and methods for KPI reporting are developed, training and change management would be delivered to clinicians to support their implementation and incorporate	1-2 years

Action	Implementation	Timeframe
	written treatment and survivorship plans into existing practices and processes.	
1.4 Develop complex referral support tools	1.4.1 Patient support organisations and blood cancer clinicians nationally to collaborate to develop a positively-oriented service directory where specialists in blood cancer subtypes are more easily identified and expected fees are outlined to support an individual's planning.	2-3 years
OBJECTIVE: Enable patient-centred care through patient reported outcomes		
1.5 Develop a national system for PROs in blood cancer	1.5.1 Given the above evidence of benefit, and the need for a nationally coordinated approach, the National Action Plan recommends assembling a clinical advisory working group of clinicians and patients by blood cancer subtype to undertake a PROs scoping study: • Define the principles and objectives of a PRO system • Agree a taxonomy for data, standards and methods of collection and analysis • Select PRO measures and identify thresholds for clinical action • Stock-take of existing clinical systems capabilities by jurisdiction • Develop a plan for implementation that ensures embedding in existing systems of care and Electronic Medical Records (EMRs).	2-3 years
OBJECTIVE: Empower and support high-risk patient groups		
1.6 Address challenges for Aboriginal and Torres Strait Islander people with blood cancer	1.6.1 In consultation with organisations managing current data collection systems (e.g. hospitals, state cancer registries, AIHW), commission an epidemiological and health services implementation research study to better understand limitations, improve data collection and statistics on blood cancers and to enable culturally sensitive care that improves primary health attendance and hospitalisation rates. 1.6.2 Work with the Aboriginal and Torres Strait Islander Health Workforce to undertake a gap review of Indigenous Health Workforce expertise in blood cancers to support culturally sensitive care across all healthcare settings (both Indigenous and non-Indigenous). 1.6.3 Develop an awareness and education campaign regarding blood cancer signs and symptoms in partnership Community Controlled Health Services. 1.6.4 Promote the use of Cancer Australia's OCP for Aboriginal and Torres Strait Islander people with cancer within the blood cancer clinical network, in conjunction with individual OCPs for different blood cancer subtypes. 1.6.5 Ensure access for Indigenous people to chemotherapy, emerging therapies and safe and effective stem cell transplants. Work with the Enabling Access Working Group (Action 4.3) and existing reform agendas underway with government and non-government organisations to increase the representation of Aboriginal and Torres Strait Islanders on the Australian Bone Marrow Donor Registry. 1.6.6 Leverage existing activities and national reform agendas to improve participation rates of Indigenous cancer patients in clinical trials for blood cancers. 1.6.7 Ensure relevance and use of supportive care assessment tool to support Aboriginal and Torres Strait Islander people living with blood cancers. The supportive care assessment tool should be validated for use with Aboriginal and Torres Strait Islanders, consistent with the advice in the OCP for Aboriginal and Torres Strait Islander people with cancer.	3-5 years
1.7 Address challenges for Culturally and Linguistically Diverse people with blood cancer	1.7.1 Ensure access to bone marrow donors for Australians of all ethnicities. 1.7.2 Undertake a stock-take of culturally and linguistically diverse patient needs by hospital to enable prioritisation by location. 1.7.3 Provide education and training to translators, cultural advocates, and local cultural organisations in blood cancer OCPs and medical terminology at high priority centres. 1.7.4 Explore options for enhanced telemedicine and digital health information services, such as the development of Royal District Nursing	1-2 years

Action	Implementation	Timeframe
	Service Talking Books and/or the National Ageing Research Institute Moving Pictures initiatives for blood cancer. This could have benefits for other patient populations, not just those from culturally and linguistically diverse backgrounds. 1.7.5 Ensure PRO systems are culturally appropriate and available in languages other than English, and the Blood Cancer Information Strategy considers culturally appropriate and accessible information provision.	
1.8 Address challenges for people with blood cancer living in regional and remote areas	1.8.1 Reduce unwarranted variation in treatment and care through development, promotion and implementation of individual OCPs for different blood cancer subtypes, clinical guidelines and accreditation, underpinned by patient navigation services, Blood Cancer Information Strategy and a workforce change management strategy. 1.8.2 Develop guidelines for diagnostics and review Australia's capacity to meet those guidelines, including the availability of specialised diagnostic services for regional and remote patients, and recommend options for improving test accuracy and appropriate guidelines for timeliness for regional and remote patients. 1.8.3 Conduct a skills audit of the regional and remote workforce and develop a strategy to enable care closer to home, adoption of tele-health, more effective diagnosis and referral of patients to subtype specialists and increased clinical trial participation in the regions. The skills audit and strategy should be done in consultation with relevant stakeholders and leverage existing work that is underway. 1.8.4 Improve opportunities to access clinical trials for blood cancer patients living in regional and remote areas, in particular through contributing to the tele-trials and the removal of barriers to travel, through advocacy and leveraging work that is already underway through the national reform agenda in clinical trials. 1.8.5 Enable patient-centred care through a national system for PROs that can enable improved monitoring of symptoms and adverse events, as well as a more systematic screening and referrals to supportive care.	3-5 years
1.9 Meet the needs of patients across their lifespan	1.9.1 The development and implementation of each action in the National Action Plan should consider the unique needs of patients across their lifespan, including palliative and end of life care. Specific considerations for life stages and circumstances must therefore be included in the OCPs and clinical guidelines, the development of the Blood Cancer Information Strategy and patient navigation services, and a national system for PROs. It will also rely on implementation at the workforce level, through training and change management, aimed at improving the understanding of blood cancers, availability of new tools and models for shared care. Consideration should be given to existing work that is already underway in order to complement, not duplicate, efforts to improve outcomes for blood cancer patients.	3-5 years

Table D.2: Actions to Achieve Best Practice

Action	Implementation	Timeframe
OBJECTIVE: Develop national standards for quality and safety		
2.1 Develop OCPs and clinical guidelines for all major blood cancer subtypes	2.1.1 Undertake a stock-take of current OCPs and national/international clinical guidelines to inform and prioritise the adoption or development of new OCPs and clinical guidelines (or update existing OCPs and clinical guidelines) for all major blood cancer subtypes. 2.1.2 Clinical guidelines would need to be updated at agreed intervals and presented at key meetings, such as the annual ALLG meeting, and endorsed by relevant professional colleges. The publication of OCPs and clinical guidelines should be considered in the development of the Blood Cancer Information Strategy, with training and education provided to support their integration and implementation into clinical practice. 2.1.3 Through the guideline development, a limited number of selected, highly specialised services may be identified as requiring high case	1 year

Action	Implementation	Timeframe
	volumes to ensure safety and quality. It is recommended that these highly specialised services are then required to be accredited via processes that are complementary to and not duplicative of existing accreditation requirements and are in line with international best practice.	
OBJECTIVE: Value an accurate diagnosis		
2.2 Develop guidelines for diagnostics and review Australia's capacity to meet these guidelines	2.2.1 Review existing international diagnostics guidelines in blood cancer and develop Australian-specific guidelines for minimum and recommended testing requirements for different types of blood cancer in Australian healthcare settings. These guidelines should be integrated with clinical guidelines (Action 2.1). 2.2.2 Following the development of diagnostic guidelines for different types of blood cancer, undertake a wider strategic assessment of blood cancer diagnostics service delivery across Australia. This would include workforce development needs in metropolitan and regional areas, as well as potential options for improving accuracy, timeliness and efficiency in diagnostic services nationally. This strategic assessment should also identify options to address issues with under-notification of cases to state cancer registries. 2.2.3 The Enabling Access Working Group (Action 4.3) should engage with governments, regulators and the blood cancer community to: a. Coordinate evidence to support the development of applications for MBS reimbursement of diagnostics that are standard of care but are not yet listed b. Continue important reforms to MSAC processes for MBS listings, focusing on greater transparency and the rapid adoption of diagnostics, which have been demonstrated to be cost-effective that direct patients to the most effective therapies. This should include enhancing consumer understanding of and engagement with the MBS listing process, drawing experience from improved consumer engagement in PBS processes.	1 year
2.3 Make precision medicine the standard of care	2.3.1 The implementation of precision medicine as a standard of care could be developed and funded as part of the Blood Cancer Research Program and supported by a working party focussed on the harmonisation of research efforts and evidence development for regulatory approval across Australia.	2-3 years
OBJECTIVE: Ensure access to supportive care		
2.4 Screen for supportive care needs	2.4.1 Screening for supportive care must be integrated into clinical guidelines to support their implementation in both primary and specialist healthcare environments. To promote uptake and use, KPIs for supportive care screening should be developed to foster improvements in clinical practice. 2.4.2 OCPs and clinical guidelines for blood cancer should recommend routine screening of patients at key milestones throughout treatment, supported through the introduction of a national system for PROs. 2.4.3 To foster the consistent screening for supportive care in clinical practice, the need for supportive care screening should be included in training and change management strategies and audited over time.	1-2 years
2.5 Improve referrals and equitable access to cancer patient-friendly exercise programs and lifestyle services	2.5.1 To improve uptake of exercise recommendations for people living with blood cancer, physical activity levels should be consistently screened as part of a supportive care screening strategy and supported by a national system for PROs. 2.5.2 Depending on the outcomes of the patient engagement and understanding of patient goals, clinicians should refer people to a program for cancer patient-friendly exercise and lifestyle services, depending on their individual support requirements. The program should trial referrals to a range of supervised and self-directed programs in both health care provider and community settings activity levels and in turn quality of life and survival.	3-5 years
OBJECTIVE: Invest in workforce development		

Action	Implementation	Timeframe
2.6 Invest in clinical capability in both primary and specialist settings	2.6.1 Define a service standard, informed by OCPs, clinical and diagnostic guidelines and the Research Roadmap, to determine workforce needs in primary and specialist settings including supportive care. 2.6.2 A skills audit of regional and remote workforce requirements. 2.6.3 Appropriate training and change management approaches developed with relevant professional bodies in primary care settings, aimed at improving the awareness and understanding of blood cancers, the availability of new tools, and models for shared care (including telehealth). 2.6.4 A training and change management strategy aimed at specialist settings, focused on advances in tele-health, personalised medicine and implications for clinical practice, the roll-out of hospital training, and the use of supportive care and palliative care for people living with blood cancer.	3-5 years
OBJECTIVE: Prevent financial hardship		
2.7 Implement reforms to address sources of financial hardship	In addition to screening for financial distress using a national system for PROs in blood cancer, supporting complex referrals for informed financial consent and patient choice, and the development of a Blood Cancer Information Strategy, the National Action Plan recommends the following reforms to prevent financial hardship: 2.7.1 Consult with all jurisdictions to reform PATS, including advocating for streamlined administrative processes, greater access to the schemes and support for patients to participate in clinical trials. 2.7.2 Review options for the introduction of financial support mechanisms (such as a temporary disability payment) to support people with cancer and other serious illness who require temporary financial support.	3-5 years

Table D.3: Actions to Accelerate Research

Action	Implementation	Timeframe
OBJECTIVE: Identify priority areas for research funding that deliver greater impact		
3.1 Identify priority areas for research funding that deliver greater impact through a Blood Cancer Research Program	3.1.1 Develop a Research Roadmap for blood cancers, with a virtual network of Australian research partners organised around disease goals or technology strengths and with business models for streamlining and augmenting collaboration across research nodes of excellence nationally and internationally. 3.1.2. The research initiatives to be prioritised should seek funding through the existing mechanisms operating for the NHMRC and MRFF, and leverage this funding, and/or seek new funding through venture capital, non-government organisations, philanthropic organisations and public-private partnerships. Where appropriate, representation should be made to government agencies to fund blood cancer priority areas, ensuring funding has the greatest impact.	3-5 years
OBJECTIVE: Harness benefits of real world data		
3.2 Improve value and use of real world data for blood cancer patients	3.2.1 The Federal Government has recognised the importance of these datasets with the National Clinical Quality Registry Strategy, which seeks to improve the value and sustainability of a range of clinical registries nationally. The Strategy is an opportunity to build data linkage, integration and interoperability capability. Through the implementation of the Strategy, a review should be undertaken to evaluate options to improve the value and sustainability of blood cancer registries and Australian blood cancer patient data by extending the coverage of these registries, leveraging from, and linking to, existing administrative datasets and annotated samples stored at related biobanks, and enabling linkages with international real world data endeavours.	3-5 years

Table D.4: Actions to Enable Access

Action	Implementation	Timeframe
OBJECTIVE: Increase access to clinical trials		
4.1 Pilot key performance indicator for discussion of clinical trial options with patients	4.1.1 The National Action Plan recommends commissioning a pilot study to examine the implementation of a KPI for a clinician-led discussion regarding enrolment in clinical trials if and where available and appropriate. A KPI for a clinical trials discussion could be piloted in blood cancers (or a subset of blood cancer patients). The results generated may have wider application for cancer patients more broadly and on a national level. The aim is to increase active consideration of clinical trials in treatment planning and empower patients to have greater engagement and understanding of their clinical trial options. 4.1.2 The establishment of a KPI for a discussion of available clinical trial options with patients would leverage existing national clinical trial reform agendas and be led by the Australian Commission on Safety and Quality in Health Care as part of their existing program of work. Once the systems and methods for KPI reporting are developed, these would be embedded in blood cancer OCPs and clinical guidelines and there would be training and change management delivered to clinicians to support their implementation.	1-2 years
4.2 Increase access to clinical trials in regional and remote areas, including a national approach to prioritising tele-trials	4.2.1 Ensuring that blood cancer OCPs and clinical guidelines document the importance of discussing clinical trial research options with patients, including those living in regional and remote areas. 4.2.2 A national approach to blood cancer research supported by the Blood Cancer Research Program, with regional trial sites pre-approved for ethics and governance to streamline trial opening, leveraging work already underway through the national reform agenda in clinical trials. 4.2.3 A skills audit of regional and remote workforce requirements (including the primary care workforce) and an infrastructure audit of facilities to enable clinical trial participation at these sites.	2-4 years
OBJECTIVE: Harmonise evidence development and regulatory applications		
4.3 Establish an Enabling Access Working Group to improve equitable access to novel and specialised therapies	4.3.1 A multidisciplinary Enabling Access Working Group should be established, including consumers, to work across the blood cancer community and address challenges for patient access to novel and specialised therapies. The Working Group would have three specific tasks: • Develop a short-list of clinically important medicines and diagnostics that do not have public subsidy and where there are market barriers to evidence development. Work with the Federal Government and the blood cancer community to coordinate an approach to evidence development for each therapy, which could include funding investigator-led clinical trials, or coordination of research and regulatory applications, including provisional registration which may require participation in a registry to enable access to a novel therapy. • Commission a review of access to novel and specialised therapies by state and territory to identify disparities in access to standard of care therapies and develop a plan to improve equity of access nationally. • Engage with government to develop a strategy to optimise supply of suitable stem cell donors for Australian and international patients and to ensure equity of access to cellular and emerging therapies, including CAR T-cells for all Australians	3-5 years

In focus: National Health Reform Agreement

The NHRA is an agreement between the Federal Government and all state and territory governments which commits to improving health outcomes for Australians, by promoting continuous care in the community, and ensuring the future sustainability of Australia's

health system. It is the key mechanism for the transparency, governance and financing of Australia's public hospital system.[85]

It is through this agreement that the Federal Government contributes funds to the states and territories for public hospital services, including for services delivered through emergency departments, hospitals and community health settings.

An Addendum and Roadmap were released in 2021 to support achievement of the following goals:

- Improving efficiency and ensuring financial sustainability
- Delivering safe, high-quality care in the right place at the right time
- Prioritising prevention and helping people manage their health throughout their lifetime
- Driving best practice and performance using data and research.

Key of reform specified in the Roadmap include:[86]

- Nationally cohesive Health Technology Assessment
- Paying for value and outcomes
- Joint planning and funding at a local level
- Empowering people through health literacy
- Prevention and wellbeing
- Enhanced health data
- Interfaces between health, disability and aged care systems.

Table D.5: Key components of NHRA

National health reform agreement commitments	Deliverable	Start	End
Agree consistent process for assessing and funding highly specialised therapies under the NHRA	Endorsed process	21-22	
Establish process to facilitate a cohesive approach to a HTA nationally	National committee	21-22	
Develop a national HTA framework, including processes to inform implementation, investment and disinvestment opportunities at Commonwealth and state levels	National framework	21-22	22-23
Establish an information sharing platform	Information sharing platform	22-23	24-25
Produce public and stakeholder guidance	Guidance materials	22-23	24-25
Identify HTA workforce requirements and develop workforce framework	Workforce action plan	22-23	24-25
Identify and prioritise technologies that will benefit from national level HTA	Agreed priority list	21-22	24-25

Source: NHRA, 2021, Long-term Health Reforms Roadmap.

[85] Department of Health website, available: https://www.health.gov.au/our-work/2020-25-national-health-reform-agreement-nhra.
[86] NHRA, 2021, Long-term Health Reforms Roadmap.

In focus: Zimmerman Report

The inquiry reviews topics to ensure that Australia continues to be well positioned to access new drugs and novel medical technologies in a timely manner and respond to emerging global trends, including:

- Range of new drugs and emerging novel medical technologies in development
- Incentives to research, develop and commercialise new drugs and novel medical technologies for conditions where there is an unmet need, in particular orphan, personalised drugs and off-patent that could be repurposed
- Measures that could make Australia a more attractive location for clinical trials
- Without compromising the assessment of safety, quality, efficacy or cost effectiveness, whether the approval process for new drugs and novel medical technologies, could be made more efficient, including through greater use of international approval processes, greater alignment of registration and reimbursement processes or post market assessment.

The Committee's recommendations are identified in the table below.

Table D.6: Recommendations

Number	Who	What
1	Federal Government	Establish a Centre for Precision Medicine and Rare Diseases within the Department of Health
2		The Health Technology Assessment (HTA) process for cell and gene therapies be simplified to establish a clear and certain pathway for such therapies
3	Federal Government	Establish an Office of Clinical Evaluation within the Department of Health to assess the best and most effective care for patients in the context of new and emerging health technologies
4		The assessment process for the Life Saving Drugs Program (LSDP) be streamlined and delays in access to treatments be reduced by ensuring that a sponsor only need lodge one application for one Health Technology Assessment pathway
5	Federal Government	Develop a labour market and skills strategy to expand the number of health economists in Australia
6	Department of Health	Increase its efforts to educate and engage with patients, clinicians, industry and the public and develop education campaigns on all aspects of the regulation and reimbursement system Improve information available on the websites of the Therapeutic Goods Administration (TGA) and its Health Technology Assessment (HTA) bodies for all users including patients, clinicians, industry and the public
7	Department of Health and National Blood Authority	Reform the Health Technology Assessment processes for blood products to provide better alignment with the Health Technology Assessment system
8	Federal Government	Changes to submission fees for the Therapeutic Goods Administration (TGA), the Pharmaceutical Benefits Advisory Committee (PBAC) and Medical Services Advisory Committee (MSAC) assessments
9	Federal Government	Establish a fund to support patients, clinicians and non-profit organisations to sponsor registration and reimbursement applications where there is no realistic prospect of a company serving as sponsor, and where the Department of Health is otherwise supportive of the application

Number	Who	What
10	Federal Government	Amend the National Health Act 1953 (Cth) to give the Pharmaceutical Benefits Advisory Committee the power to authorise Managed Access Programs
11	Department of Health	Conduct a comprehensive consultation process with industry to establish a more flexible way forward for the repurposing of drugs in Australia
12	Therapeutic Goods Administration	Make changes to its Orphan Drugs Program
13	Department of Health	Reform its regulatory and reimbursement processes to enable therapeutic goods to be registered and reimbursed by molecular indication in addition to by disease indication
16	Department of Health	Investigate further opportunities for the formation of an international Health Technology Assessment consortium similar to the Access Consortium to streamline the regulatory process for certain medicines and medical technologies
18	Department of Health	Conduct a review of the National Immunisation Program
20	Federal Government	Establish a last resort mechanism for directly securing ongoing supply of medicines that meet a high clinical need and lack suitable alternatives that are at risk of being delisted from the Pharmaceutical Benefits Scheme
22	All levels of government	Prioritise and implement with urgency the harmonisation of Human Research Ethics Committee (HREC) and Site-Specific Assessment submissions into one Australian online platform and enable parallel review by HRECs and Research Governance Offices
23	All levels of government	Provide funding for the development of a national clinical trial register
24	Federal Government	Develop policies that encourage modernising digital technologies and practices to position Australia as the premier destination for international clinical trials
25	Federal Government	Develop a national standard approach, including nationally agreed systems and standard operating procedures to support and strengthen the capacity to conduct clinical tele-trials in rural, regional and remote areas
26	Federal Government	Continue to fund Clinical Trial Networks with a particular focus on developing seed funding for Indigenous Health Clinical Trial Networks
27	Federal Government	Reform data exclusivity provisions in Australia with a view to extending data exclusivity for orphan drugs and vaccines to a period of up to 10 years
28	Department of Health	Integrate the patient voice upfront into the Health Technology Assessment system Implement a notification system for all HTA bodies and the TGA to advise relevant patient groups of the receipt of an application Provide patients and stakeholders with a concise sponsor's submission summary Consider making patient evidence compulsory for certain applications, and should consider the role of patient evidence in the decisions of the Therapeutic Goods Administration Notify relevant patient groups of the outcome of the assessment process by all HTA bodies
28	Federal Government	Provide funding for organisations to support participation in the HTA process, including for very rare disease patient groups that have limited capacity for fundraising or access to alternative funding
29	Federal Government	Amend the National Health Act 1953 (Cth) to formalise the role and powers of the Pharmaceutical Benefits Advisory Committee Executive

Number	Who	What
29	Department of Health	Produce a pre-submission advice framework for submissions to the Therapeutic Goods Administration, Pharmaceutical Benefits Advisory Committee, Medical Services Advisory Committee and other Health Technology Assessment bodies, explaining the interaction between those bodies and their evidentiary and other requirements, to be provided to sponsors before they make their submissions
29	Independent Health Technology Assessment Review	Reassess relevant aspects of the Health Technology Assessment process to ensure there are future pathways for treatments and therapies that do not fit neatly into the current system such as rare cancers, antimicrobials, orphan drugs, and precision medicines
29	Department of Health	Publish data on application processing times and positive recommendation rates for the Pharmaceutical Benefits Advisory Committee and other Health Technology Assessment bodies
29	Federal Government	Develop a suite of clear and measurable benchmarks to track the Commonwealth's implementations of the recommendations made by the Committee and accepted by the Federal Government
30		Per para 11.31 of Zimmerman Report
31		Per para 11.32 of Zimmerman Report

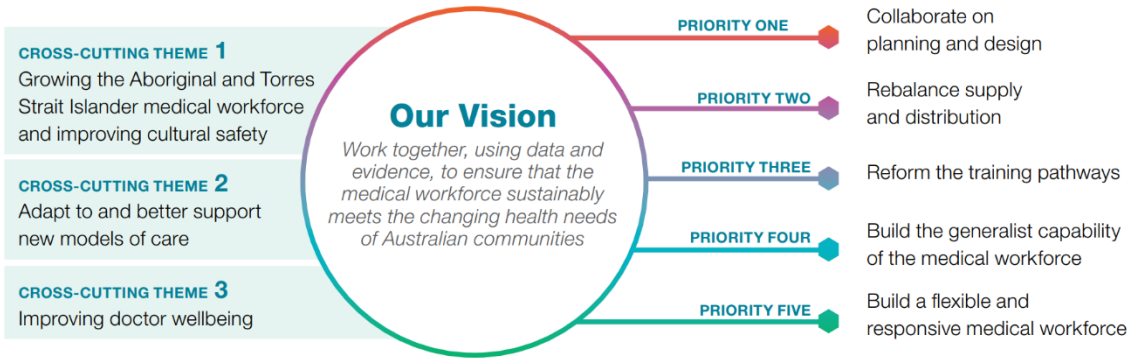
Source: Commonwealth Government, 2021, Inquiry into approval processes for new drugs and novel medical technologies in Australia.

This inquiry currently has not yet received a response from the Federal Government.

In focus: National Medical Workforce Strategy

The National Medical Workforce Strategy is a vision for using data and evidence to develop and maintain a high-quality, effective, and well-distributed medical workforce. It focuses on doctors, not the full health care team.

Figure D.1: Priorities of the National Medical Workforce Strategy



In focus: Primary Care 10 Year Plan

The plan identifies 12 action areas that are grouped under 3 reform streams:[87]

1. Future focused primary health care.

   A. Support safe, quality telehealth and virtual health care.

   B. Improve quality and value through data-driven insights and digital integration.

[87] Australian Government, 2022, Future focused primary health care: Australia's Primary Health Care 10 Year Plan 2022-2032.

C. Harness advances in health care technologies and precision medicine

2. Person-centred primary health care supported by funding reform.

   A. Incentivise person-centred care through funding reform.

   B. Boost multidisciplinary team based care.

   C. Close the Gap through a stronger community controlled sector.

   D. Improve access to primary health care in rural areas.

   E. Improve access to appropriate care for people at risk of poorer outcomes.

   F. Empower people to stay healthy and manage their own health care.

3. Integrated care, locally delivered.

   A. Joint planning and collaborative commissioning.

   B. Research and evaluation to scale up what works.

   C. Cross-sectoral leadership.

In focus: Draft Australian Cancer Plan

Aim of draft Australian Cancer Plan:

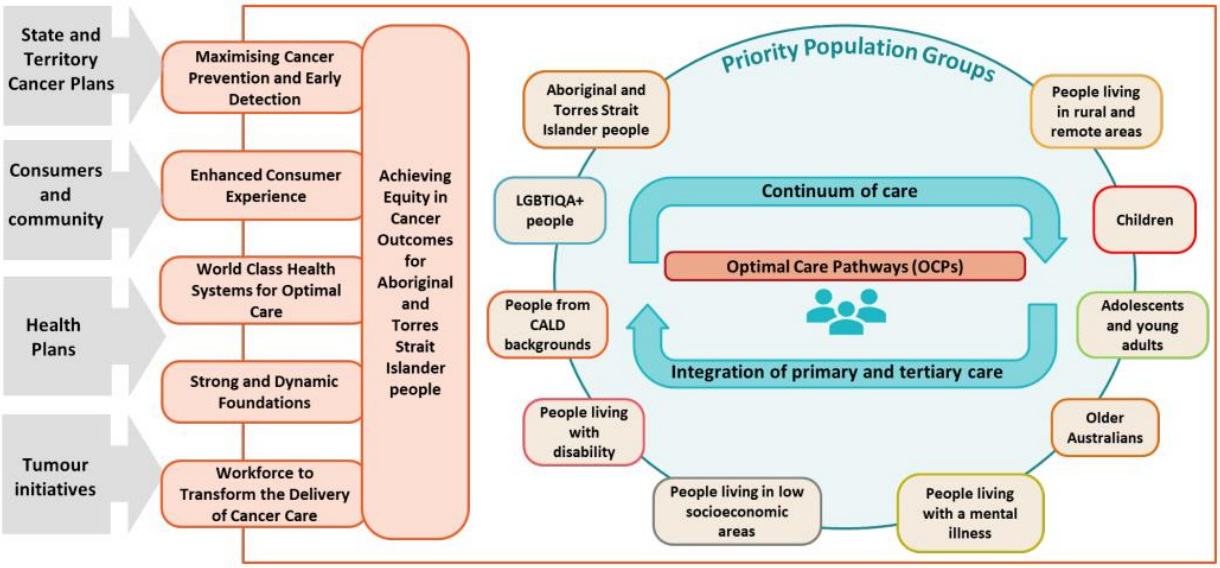
> The future-focused, ten-year Australian Cancer Plan (ACP) is a national framework that will accelerate world class cancer outcomes and improve the lives of all Australians affected by cancer

Vision of draft Australian Cancer Plan:

> World-class cancer outcomes and experiences for all Australians.

The conceptual framework (below) which supports the vision of the draft Australian Cancer Plan is underpinned by cancer-specific OCPs and by the Optimal Care Pathway for Aboriginal and Torres Strait Islander people with cancer.

Figure D.2: Australian Cancer Plan – Conceptual framework



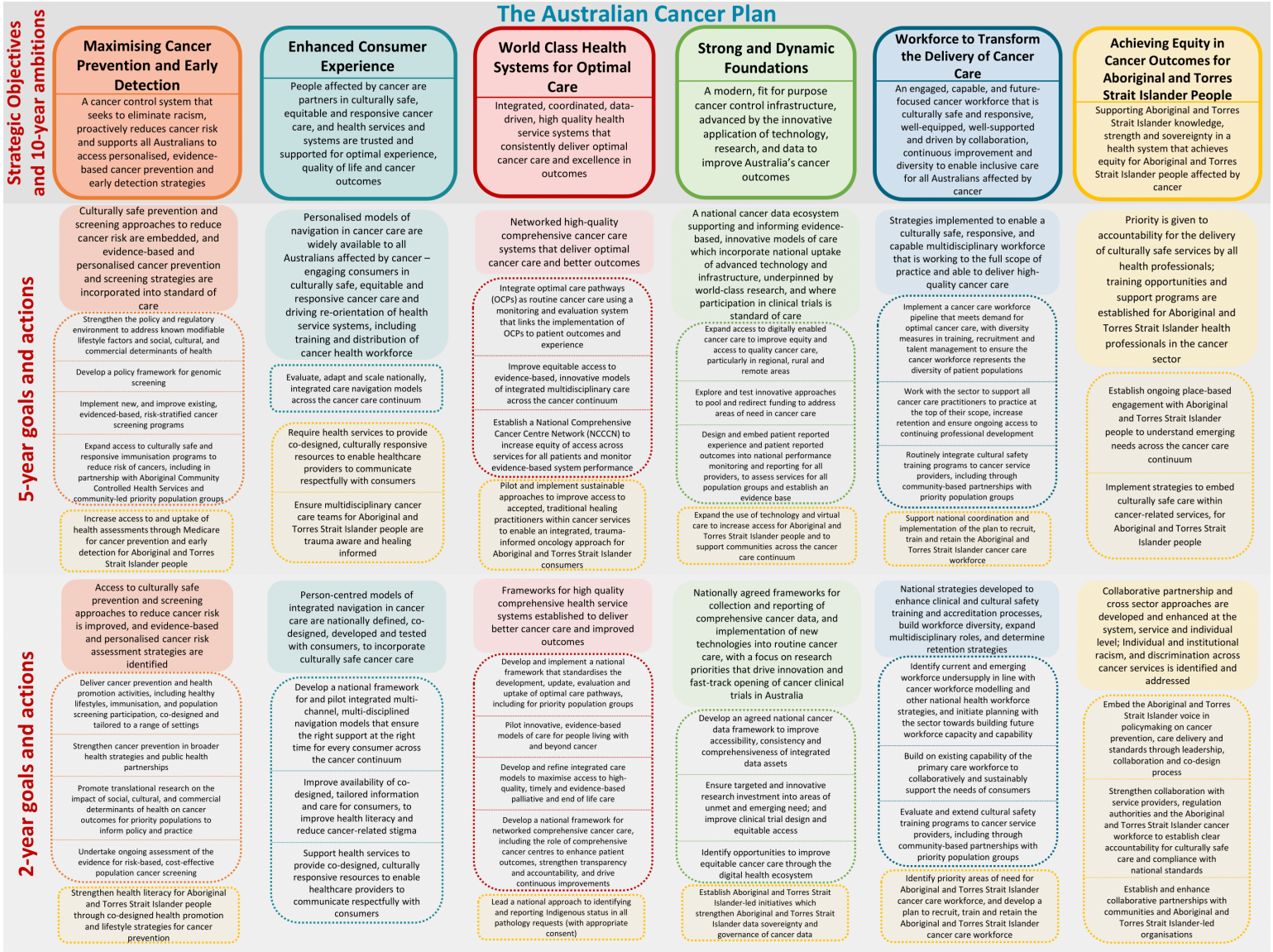
Source: Australian Cancer Plan, 2022, Draft ACP Summary.

The draft 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.

The associated 10, five, and two year actions and goals are summarised overleaf.

Figure D.3: Australian Cancer Plan – Plan on a page



In focus: HTA Review

The HTA Review is to consider a number of important areas including:[88]

- Selection of comparator(s)
- Methods for evaluating rare diseases for reimbursement and alternative funding pathways if required
- Methods for evaluating new and emerging technologies (including cell and gene therapies, and other precision-based medicines) and the suitability of existing funding pathways as required
- Methods for evaluating all new medicines and vaccines
- Use of real-world evidence including from sources other than randomised controlled trials
- Managing clinical, economic, financial and other uncertainty
- The feasibility of international work-sharing for reimbursement submissions.

In focus: National One Stop Shop and National Clinical Trials Front Door

Commissioned by the Commonwealth Government, the Australian Commission on Safety and Quality in Health Care is developing a National One Stop Shop and National Clinical Trials Front Door aiming to make it easier for patients, researchers, industry representatives and sponsors to find, conduct, participate and invest in high quality and ethical research in Australia.[89] It will:

- Provide a cross-jurisdictional ethics approval and site-specific authorisation platform that incorporates key application, notification and approval systems
- Incorporate the Clinical Trials Notification and Clinical Trials Approval schemes administered by the TGA
- Include an embedded and automated next-generation national clinical trials registry
- Provide sophisticated monitoring and reporting functionality for different users.

Additionally, the National One Stop Shop will:[90]

- Embed the National Clinical Trials Governance Framework accreditation obligations and automate data/reports/processes to support the accreditation process
- Assist all governments to respond to areas of need in a rapid, coordinated and strategic manner based on real-time, accurate information regarding trial activity and site capability
- Aim to extend beyond the public and private hospital sector to incorporate the university, primary care and independent medical research sectors
- Protect data integrity and adhere to data security requirements.

[88] Department of Health, 2022, Health Technology Assessment Policy and Methods Review Frequently Asked Questions Page.
[89] https://www.safetyandquality.gov.au/our-work/health-and-human-research/national-one-stop-shop-national-platform-health-related-human-research
[90] https://www.safetyandquality.gov.au/our-work/health-and-human-research/national-one-stop-shop-national-platform-health-related-human-research

Appendix E
Acronyms

HSANZ	Haematological Society of Australia and New Zealand
ICD	International Classification of Diseases
KPI	Key Performance Indicator
LaRDR	Lymphoma and Related Diseases Registry
LLS	Leukaemia & Lymphoma Society
MALT	Mucosa-Associated Lymphoid Tissue
MBS	Medicare Benefits Scheme
MCL	Mantle cell lymphoma
MDS	Myelodysplastic Syndrome
MDT	Multi-Disciplinary Team
MOGA	Medical Oncology Group of Australia
MPN	Myeloproliferative neoplasms
MSAC	Medical Services Advisory Committee
NAS	National Aggregate Statistics
NCI	National Cancer Institute
NFC	Nationally Funded Centres
NHL	Non-Hodgkin Lymphoma
NHMRC	National Health and Medical Research Council
NHS	National Health Service
NICE	National Institute for Clinical Excellence
NIH	National Institutes of Health
NNDSS	National Notifiable Disease Surveillance System
NOA	National Oncology Alliance
NORD	National Organisation for Rare Disorders
NPV	Net Present Value
NZ	New Zealand
OBPR	Office of Best Practice Regulation
PARP	Poly ADP Ribose Polymerase
PBAC	Pharmaceutical Benefits Advisory Committee
PBS	Pharmaceutical Benefits Scheme

PD-1	Programmed T cell death 1
PHN	Primary Health Network
PRO	Patient Reported Outcome
PTCL	Peripheral T-cell lymphoma
QALY	Quality Adjusted Life Year
SEER	Surveillance, Epidemiology and End Results
TGA	Therapeutic Goods Administration
TKI	Tyrosine Kinase Inhibitor
UK	United Kingdom
WhIMSICAL	Waldenström's Macroglobulinemia Study In CART-WheeL
YLD	Years Lived with a Disability
YLL	Years of Potential Life Lost
ABS	Australian Bureau of Statistics
AI	Artificial Intelligence
AIDS	Acquired Immune Deficiency Syndrome
AIHW	Australian Institute for Health and Welfare
ALL	Acute Lymphoblastic Leukaemia
ALLG	Australasian Leukaemia and Lymphoma Group
AMA	Australian Medical Association
AML	Acute Myeloid Leukaemia
ANZCHOG	Australian and New Zealand Children's Haematology and Oncology Group
AYA	Adolescents and Young Adults
BCL-2	B-cell lymphoma 2
BCR	Benefit Cost Ratio
CAR T-cell therapy	Chimeric Antigen Receptor T cell therapy
CART-Wheel	Centre for Analysis of Rare Tumours
CDC	Centers for Disease Control and Prevention (US)
CLL	Chronic Lymphocytic Leukaemia
CML	Chronic Myeloid Leukaemia
CNS	Central Nervous System
COSA	Clinical Oncological Society of Australia
CRO	Contract Research Organisation
CTCLs	Cutaneous T-cell lymphomas
ctDNA	Circulating tumour DNA
CTU	Clinical Trial Unit

DALY	Disability Adjusted Life Year
DLBCL	Diffuse large B-cell lymphoma
DNA	Deoxyribonucleic acid
EMA	European Medicines Agency
ESMO	European Society for Medical Oncology
EU	European Union
FDA	Food and Drug Administration
FL	Follicular lymphoma
GP	General Practitioner
HIV	Human Immunodeficiency Virus
PROs	Patient Reported Outcomes
SoTN	State of the Nation
NAP	National Action Plan
ACP	Australian Cancer Plan
CPMRD	Centre for Precision Medicine and Rare Diseases

Appendix F
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