A Rare Cancer Moonshot

Equity for everyone with cancer

rare cancers Australia

A moonshot

A moonshot is an ambitious, exploratory, and ground-breaking project undertaken without expectation of near-term profitability. The term is inspired by the Apollo 11 moon-landing project.

Key aspects of a moonshot include:

Radical solution: It aims to solve a huge problem or create a revolutionary change.

High-risk: The stakes are high, but the potential rewards are enormous.

Innovation: It often involves developing new, cutting-edge technologies.

Long-term perspective: Moonshots typically require immediate investment, dedication and continued effort in return for long-term transformation.

Sponsors

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Rare Cancers Australia

At Rare Cancers Australia (RCA), our mission is to improve the lives and health outcomes of people affected by rare, less common, and complex cancers. We tackle individual challenges through comprehensive patient support and address systemic issues through dedicated policy and advocacy. We believe that no one should face their cancer journey alone. Our commitment extends to driving change in access, affordability, and quality of care, ensuring better outcomes for the patients of today and tomorrow. We aim to be a beacon of hope, a force for change, and a steadfast ally for people living with rare and less common cancers.

rare cancers Australia

This report is dedicated to all the people we have supported and learned from over the last 12 years, in particular RCA co-founder Kate Vines, who 30 years on still lives with a stage 4 rare cancer, for which there is still no standard of care. For everyone who lives with a rare cancer, for their families and for those who couldn't wait any longer, we will continue to shoot for the moon until things are fair for you.

Thanks to the RCA team who bring their passion to our important work every day. Special acknowledgement to the talented authors of this report Sarah Benger, Shawn Clackett and Plum Stone.

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Acknowledgement of Country

Rare Cancers Australia acknowledges the Aboriginal and Torres Strait Islander peoples as the Traditional Custodians of the lands and waters on which we all work, live, and learn. We honour and celebrate the rich cultural heritage, strength, and resilience of the world's oldest living cultures, including traditional practices, diverse languages, and deep spiritual connection to Country.

We recognise the immense importance of connection to land and community for Aboriginal and Torres Strait Islander peoples. This bond forms the foundation of identity, wellbeing, and cultural continuity. The strength and resilience of Indigenous communities, despite historical wrongs, is a testament to the power of these connections.

We also acknowledge the significant disparities in cancer outcomes experienced by Aboriginal and Torres Strait Islander peoples, who are more likely to be diagnosed at an advanced stage, face multiple co-morbidities, and experience delays in starting treatment. Culturally uninformed healthcare services, including language barriers and a lack of understanding of Indigenous perspectives on cancer, significantly influence experience and outcomes.

The Australian Cancer Plan recognises Aboriginal and Torres Strait Islander peoples as a priority population due to the disproportionate incidence and unacceptable gap in survival compared to non-Indigenous Australians.[1, 2] We are committed to working with communities and within the frameworks of the National Agreement on Closing the Gap and the Aboriginal and Torres Strait Islander Cancer Plan to improve outcomes and experience.

Aboriginal and Torres Strait Islander communities continue to demonstrate incredible strength and resilience. A deep understanding of holistic health and wellbeing, which encompasses physical, emotional, spiritual, and cultural dimensions, coupled with the strength of community and connection to Country, provides a powerful foundation for addressing the challenges of cancer.

We recognise the importance of culturally safe and responsive healthcare services that respect and incorporate Indigenous knowledge and perspectives. By working together, we can build on the strengths and resilience of these communities to improve cancer outcomes and support the health and wellbeing of all Aboriginal and Torres Strait Islander peoples.

We pay our respects to Elders past and present, and extend that respect to all Aboriginal and Torres Strait Islander peoples.

Foreword

Every day at Rare Cancers Australia, we hear from patients navigating a healthcare system that can seem as uncompassionate as it is complex.

They tell us of the immense financial burdens of services and treatments, the frustration of not being able to access vital information, and the heartache of feeling powerless to make informed decisions about their care. The cost of rare and less common cancers extends far beyond the physical toll – it profoundly impacts the emotional and financial well-being of patients, their loved ones, the community and our economy.

These challenges resonate with clinicians, supporters, and advocates across the political spectrum. Rare cancers do not discriminate. The issues have been acknowledged in Senate and House of Representatives inquiries and through the ongoing review of Health Technology Assessment processes. There is a wide variety work from many NGOs and think tanks, and we have broad recognition that change is needed, yet progress remains frustratingly slow.

We've had the conversations. We've heard the recommendations. Now is the time for decisive action.

Over the past six years at RCA, I have witnessed firsthand the incredible resilience and spirit of our community, and heard their expectation for change. I believe in the potential for transformative change when we unite behind a common purpose.

Rare Cancers Australia has a proud history of advocating for the needs of patients and their families. Stepping into the role of CEO at Rare Cancers Australia, I am filled with a sense of urgency and determination. As we look to the future, we must be even bolder and even more ambitious in our efforts.

This moonshot moment calls upon all of us – government, health care providers, industry, researchers, and the community – to come together with a shared commitment and a united voice. Together, we can create a future where no person is disadvantaged because of the rarity of their cancer. We must work tirelessly until every Australian affected by a rare or less common cancer has timely access to the information, treatments, and support they need and deserve.

Time is of the essence. Patients have waited long enough. Let's roll up our sleeves and make change happen. Together, we can turn the tide against rare cancers and build a healthcare system that is fair, compassionate, and equitable for all.

The countdown is on – join us on this critical mission.

> "Together, we can turn the tide against **rare cancers** and build a healthcare system that is fair, compassionate, and equitable for all."

Christine Cockburn
Chief Executive Officer, Rare Cancers Australia

Executive summary

In 2023, more than 51,000 people died from cancer in Australia. More than one third of these deaths were from a rare or less common cancer.[3]

Despite improvements in overall cancer survival rates, the stark inequities faced by people with rare and less common cancers persist. On average, the five-year relative survival is 62% for rare cancers and 45% for less common cancers, compared to 77% for common cancers.[3] For some rare and less common cancers, such as bladder, urethral and eye cancers, improvements in survival have slowed or even declined.[4]

Cancer affects every aspect of a person's life and has a ripple effect on those around them, their communities and our economy. Investment in new technologies, therapies, and services to extend the prognosis and quality of life of people with non-curative cancer can return $3.17 billion of social value.[5]

This is not new information. The disparities facing people with rare and less common cancers have been recognised for over a decade, yet little has changed. Resounding calls for action from patient advocates, parliamentary inquiries, and national strategies have failed to translate into meaningful improvements in experiences or outcomes for those affected by rare and less common cancers.

Incremental change is no longer enough. To realise the Australian Cancer Plan's vision of equity for all Australians with cancer,[1] we must dramatically alter the trajectory for rare cancers. Bold action and unwavering commitment are required, with efforts scaled to match the magnitude of the challenge. There is no single action that will deliver equity of outcomes and experience. It requires strategic investment, policy reform, and collective action across six priority areas, working within and extending the strategic objectives of the Australian Cancer Plan and respective state cancer plans.

Through a united '**Rare Cancer Moonshot**', we can forge a future where no one is disadvantaged due to the rarity of their cancer *(see Figure 1)*.

62%

Five-year relative survival for rare cancers on average

45%

Five-year relative survival for less common cancers on average

77%

Five-year relative survival for common cancers on average

A Rare Cancer Moonshot

demands a concerted effort across government, healthcare, research, industry, and the community to radically accelerate progress so that by 2035:

More than 90% of people diagnosed with cancer survive at least five years after diagnosis, regardless of rarity; and

There is **true equity of experience** for everyone with cancer.

Figure 1. The Rare Cancer Moonshot

RECOMMENDATIONS

Over the next year, there are six immediate priorities:

1 Deliver the Senate inquiry recommendations

The Australian Government should endorse and deliver all 41 recommendations of the Senate inquiry into Equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer.[6] There needs to be a nationally coordinated approach with states, territories, funded bodies, and the rare cancer community to implement the recommendations in full.

2 Develop and implement the National Genomics Strategy

Genomics Australia (once established) and Cancer Australia should work with the Australian Government Department of Health and Aged Care, and state and territory governments, to deliver a National Genomics Strategy and National Framework for Genomics in Cancer Control that:

- delivers comprehensive and cost-effective genomic profiling as standard of care, starting with patients who have the highest unmet need
- equips and supports all health systems to introduce genomic profiling and matched therapies as a coordinated part of cancer pathways, including the smooth transition of comprehensive genomic and functional studies methods into clinical practice.

3 Reform Australia's Health Technology Assessment (HTA) system

The Government should publish and respond to the recommendations from the HTA Review. As an immediate priority within the reform, it should implement a new pathway in HTA that:

- assesses precision oncology companion diagnostics and therapies together
- recognises limited clinical data that arise from small patient populations and therefore utilises broader value measures
- accommodates the assessment of multiple indication, genomic-focussed applications to expedite access for people with rare and less common cancers.

As an immediate measure, to ensure greater patient access while long-term improvements are put in place, the Pharmaceutical Benefits Advisory Committee (PBAC) should establish decision-making frameworks that assess pan-tumour applications for new medicines approval and prioritise applications that will broaden access to medicines already approved for different indications.

4 Invest in novel clinical trials and telehealth

Government, healthcare providers and industry partners should invest in novel clinical trial designs and telehealth infrastructure to expand access to precision oncology for rare cancers and patients in rural and regional areas.

5 Ensure rare and less common cancers are represented within the National Cancer Data Framework

Cancer Australia and the Australian Institute of Health and Welfare (AIHW) should:

- prioritise the rapid development and implementation of an ambitious National Cancer Data Framework and minimum cancer data set, building on the strengths of state registries and enabling a more timely, accurate and person-centred national view of cancer pathways and outcomes for rare and less common cancers
- work with states and territories to ensure all cancer registries are capturing timely, high-quality and consistent data across the cancer pathway, including molecular subtype and stage at diagnosis and indigenous status, for all cancer subtypes.

6 Prioritise research funding with a focus on translation into clinical practice

The National Health and Medical Research Council (NHMRC) and the Medical Research Future Fund (MRFF) should prioritise research into rare cancers, genomics, the application of artificial intelligence, innovative testing and treatment, and critically, its translation into clinical practice.

The time for incremental change has passed. Through a Rare Cancer Moonshot, we have an opportunity to mobilise the passion, expertise, and resources needed to transform rare cancer outcomes and experiences. Together, we can create a future where no one faces inequity due to the rarity of their cancer.

A better world for rare

"Since Kate and I formed **Rare Cancers Australia,** over 250,000 Australians have died from rare and less common cancers. The rapid progress in science and technology over that time means that today's patients need not suffer the same fate. Many, if not most, can now live longer and better. We know how to make this happen, we just need the will, the courage and the compassion. It's our choice."

Richard Vines, Chair and Co-Founder of Rare Cancers Australia

Remarkable progress in our understanding of cancer is driving a dramatic shift in prevention, diagnosis, treatment, and monitoring.

Genomic testing and targeted therapies offer hope; across the globe, targeted screening programs and new diagnostic tests are being refined. Immunotherapies are being developed at a rapid pace and personalised cancer vaccines are emerging, offering targeted and less toxic treatment options. There are so many reasons to be hopeful for a future free from the burden of cancer.

Yet every day, we hear from people who feel isolated, helpless, and let down as they struggle to afford cancer treatments and face agonising decisions with limited information and support. And, despite overall cancer survival rates improving, there is a stark difference between the survival rates of different cancer types and subtypes.

Australia's healthcare and support systems are not keeping pace with scientific and technological advances, denying access to accurate diagnoses and treatments that could extend and improve the quality of life of people living with rare and less common cancers. Medicines routinely subsidised for a person with a common cancer remain prohibitively priced for a person with a rare cancer.

"As cancer treatment has developed in Australia and internationally, awareness of rare and less common cancers has increased. Whilst increased awareness is welcome, the evidence to the Inquiry is clear that this has not translated to equity in outcomes for patients with these cancers compared to patients with more common cancers."

Senate Standing Committee on Community Affairs
Equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer[6]

A better world for rare is within our reach. There are game-changing technologies, new treatments and evidence-based models of holistic care and support available right now, but we must make use of them and demand the best for every person diagnosed with cancer in Australia.

We need to come together across healthcare and support systems, industry, consumer, and community groups, locally and internationally, to radically accelerate change and create a better world for rare cancers; where survival and experience are not worse due to the rarity of someone's diagnosis.

Recognition of rare

> An informed and engaged public can play a fundamental role in driving policy changes that prioritise the needs of patients with rare cancers. Advocating for legislative changes aimed at improving healthcare and treatment access, research funding and patient rights may result in a more equitable and compassionate healthcare system.
>
> Ultimately, by coming together to raise awareness about rare cancers, we can ignite a ripple effect of positive change that empowers patients, families, and communities to confront rare cancers with a level of confidence, resilience, and hope."
>
> **Emily, diagnosed with NUT carcinoma in 2019**

In 2014, Rare Cancers Australia launched its first report, *Just a Little More Time*, urgently calling for equity in outcomes and experience for people with rare and less common cancers.[7]

In 2020, the National Oncology Alliance, with more than 400 registered stakeholders, united behind a vision for a patient-driven health system that could achieve greater than 90% survival for every cancer type and subtype, regardless of rarity.[8]

The decade since *Just a Little More Time* has seen a welcome boost in the recognition of rare and less common cancers. Much needed research is being driven through the MRFF, set up in 2015, with $37.9 million invested across 25 rare cancer-specific research grants.[6, 9] The NHMRC committed $221.6 million for 186 grants relevant to rare and less common cancers between 2018 and 2022.[6] Two new 10-year MRFF Missions on low survival cancers and health services for groups who experience inequitable outcomes were launched in 2024.[10]

The long-awaited launch of Genomics Australia is another vital piece of the puzzle for rare cancers, with a vision to "ensure that Australia is a world leader in the research, development, and use of genomic medicine to save lives and protect lives" and advise on the $500 million 10-year genomics mission.[11, 12]

The release of Australia's first Aboriginal and Torres Strait Islander Cancer Plan[13] and first Australian Cancer Plan[1] recognised the specific challenges and inequities facing priority populations, with a renewed focus on equity of outcomes and experience for all Australians diagnosed with cancer.[1] The leadership and coordination provided by Australia's first national cancer plan is particularly vital for rare and less common cancers, where small populations require greater collaboration across the country and internationally to transform outcomes and experience.

Several parliamentary inquiries and reviews examining cancer, medicines approval, and the translation of research into clinical practice, have investigated the opportunities, challenges and barriers of improving outcomes.[14-16] In November 2023, the Government tabled a response to *The New Frontier – Delivering Better Health for all Australians* report.[12, 17] The inquiry into the approval processes for new drugs and novel medical technologies delivered 31 recommendations, with many recognising that current approval processes disadvantage rare cancer patients in accessing medicines and clinical trials.[18] In May 2024, the Senate inquiry into *Equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer* published its extensive report.[6] The Committee made 41 recommendations addressing inequities for people with rare and less common cancers and how to close the 'survival gap' between rare and common cancers,[6] and we await the Government's response.

Our changing understanding of rare and less common cancers

Despite the low incidence of each individual rare or less common cancer, collectively they represent over a quarter of all people diagnosed with cancer in Australia – more than any single common cancer type.

Approximately 40,000 people are diagnosed with a rare or less common cancer[a] in Australia each year, representing 27% of all cancers diagnosed and 37% of all cancer deaths.[3]

While there is European consensus that incidence is the best criterion for defining rare cancers,[19] there will be clinical presentations which are rare occurrences despite being a common tumour type, based on the characteristics. For example, breast cancer in men and all paediatric cancers.

Despite their diversity, people with rare and less common cancers face a common set of challenges, including late and protracted diagnosis, limited treatment options and uncertain prognosis, feelings of isolation and a lack of information and support.

Further, as our understanding of cancer and ability to sequence genomes has evolved, many cancers previously considered common are now identified as collections of rare subtypes. For example, lung cancer, the fifth most common cancer in Australia (after prostate, breast, melanoma of the skin, and colorectal), was once categorised as either small cell or non-small cell lung cancer. Today, we recognise over 30 subtypes of lung cancer, many of which are rare. People with those rare subtypes report similar experiences to those with tumour-site classified rare cancers, such as limited information, fewer treatment options, higher costs, and a sense of isolation.

Rare subtypes are not yet reflected in AIHW statistics, meaning the actual proportion of rare and less common cancers may be significantly underestimated. The AIHW is using exploratory cancer incidence data based on histology as a step towards a more reflective classification approach.[20] In our 2023 *Rarefication* report we recommended the AIHW include data on molecular subtypes and support the establishment of appropriate registries to adequately facilitate data collection.[21] This remains critical and we are working with AIHW on addressing the challenges of rare and less common data collection and reporting.

RARE CANCERS AUSTRALIA

Rare Cancers Australia provides support to any person with a rare, less common, or complex cancer, recognising the limitations of the rare and less common definitions. In time, we expect more cancers to become rare in presentation as the molecular subtype and other characteristics are better understood. Therefore, addressing the issues and barriers facing people with rare and less common cancers is more crucial than ever.

Since my diagnosis, it has been a whirlwind. I had never heard of rare cancers before my diagnosis, let alone fallopian tube cancer. My mother had breast cancer, so I've always been very diligent with mammograms thinking that that was where my risk was. When, in reality, there are so many other cancers out there that you just don't hear about."

Patricia, diagnosed with stage 3 fallopian tube cancer in 2024

a A rare cancer is classified as one with an incidence of less than six per 100,000 people, and a less common cancer has an incidence of between six and 12 per 100,000 people.

No one is left behind

Cancer is the leading cause of death in Australia, significantly contributing to the nation's disease burden.[1] In 2023, it was estimated that 165,000 people were diagnosed and more than 51,000 people, including more than 400 children and young adults, died from cancer.[3]

To ensure no one is left behind as we make progress against cancer, it is crucial to address the unique challenges faced by people with rare and less common cancers.

Unfortunately, there is a lack of national data capturing patient-reported experiences of their cancer care. However, there is growing consensus regarding the vastly different experiences of people with different cancer types and the inequity faced by those with rare cancers.

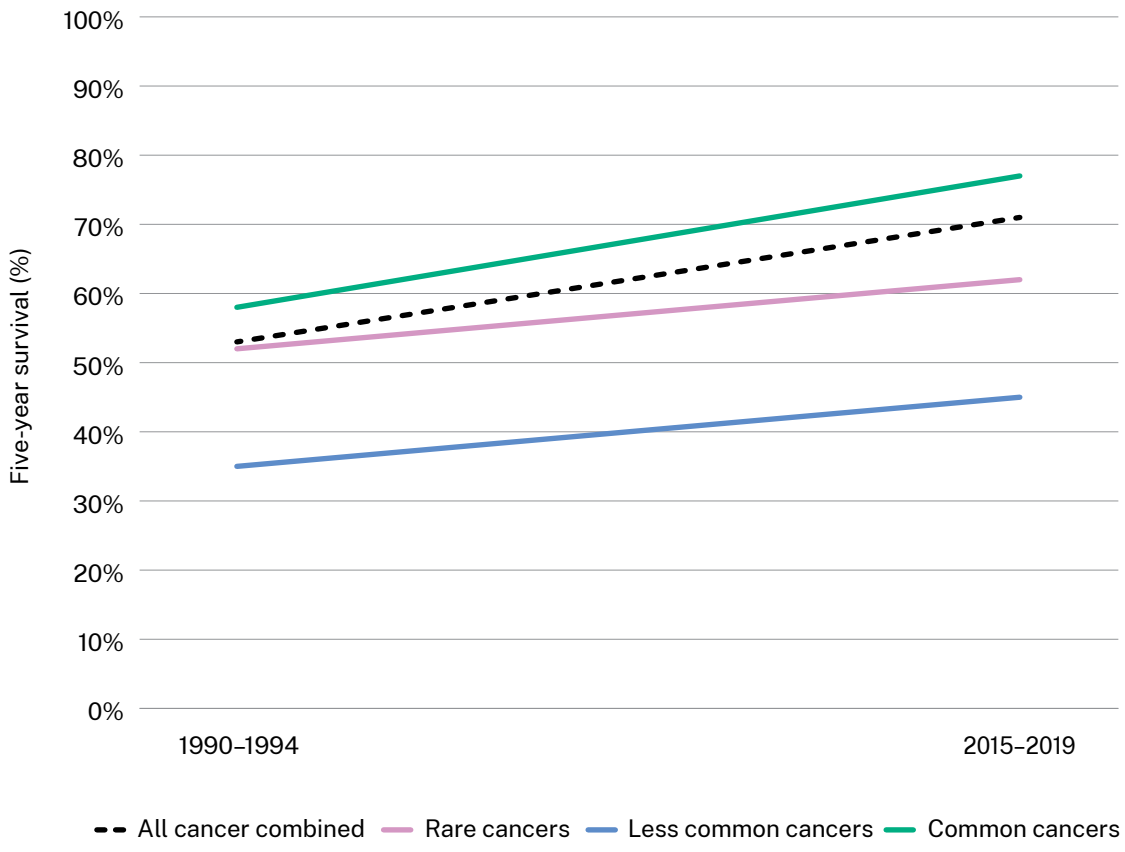
> "Whilst cancer outcomes in Australia are amongst the best in the world, this inquiry has made clear that these outcomes are not felt equally amongst all Australian cancer patients... Those diagnosed with rare or less common cancers do not always receive the same level of support or have access to the same diagnosis and treatment options, as those with more common cancers, having, at times, deadly consequences."
>
> **Senate Standing Committee on Community Affairs**
> *Equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer*[6]

Overall cancer survival is improving, but not equitably for all cancers

Australia boasts internationally strong cancer survival rates, with a steady increase over time. But focusing on overall survival masks the alarming difference in the pace of change for many cancers.

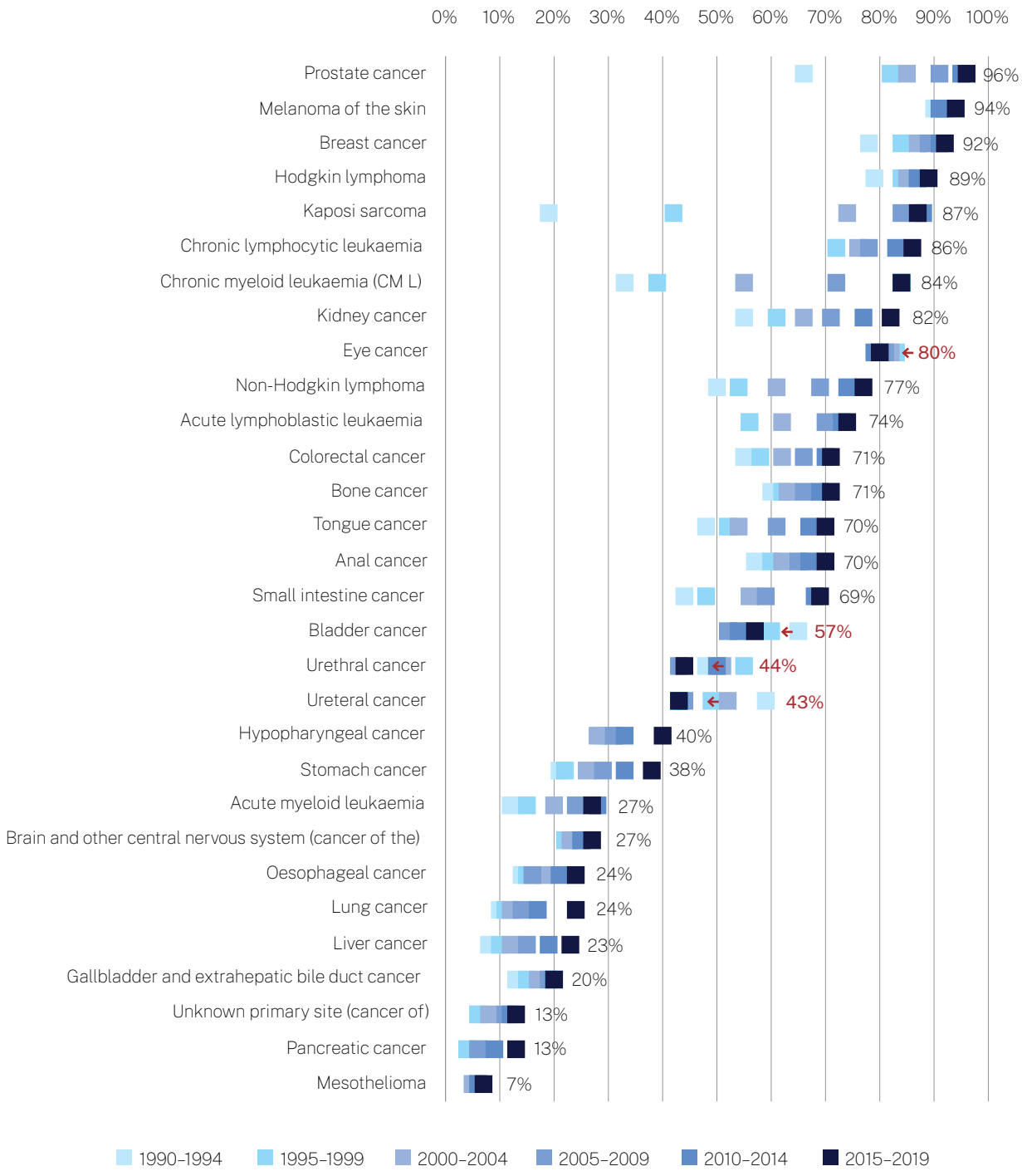
Since the 1990s, the five-year relative survival for common cancers has improved significantly more than it has for rare and less common cancers. Between 2015 and 2019, the five-year relative survival rate was 77% for common cancers, 62% for rare cancers, and 45% for less common cancers *(see Figure 2)*.[3] This disparity highlights the urgent need to focus on rare and less common cancers to ensure no one is left behind.

Figure 2: Relative five-year survival for all cancers combined over time[3]



Improvements in overall survival by cancer type present a mixed picture. Encouragingly, five-year survival has improved significantly for some rare and less common cancers such as chronic myeloid leukemia, oropharyngeal cancer, and multiple myeloma. But for others, including mesothelioma and cancers of the lip, blood, and brain, there has been little improvement.[3] In some cases, such as eye, urethral, and bladder cancers, five-year survival may have declined *(see Figure 3)*.[3]

Figure 3: Relative five-year survival over time, in select cancer types[3]



Inequity in experience and outcomes

There are significant disparities in cancer experience and outcomes between specific population groups in Australia.[22] The Australian Cancer Plan noted that these disparities are strongly associated with the social determinants of health and identified 10 priority populations requiring tailored, appropriate and adequately resourced services to meet their needs and achieve equity in care for everyone.[22]

The Australian Cancer Plan notes that Aboriginal and Torres Strait Islander peoples experience greater preventable risk factors, reduced participation in screening programs, less access to primary care and specialist services, and cultural and language barriers in the health system.[1]

Improvements in overall survival rates (*see Figure 3*) mask the stark difference in outcomes for Indigenous Australians. Cancer deaths are 1.4 times higher in Indigenous Australians than in other Australians.[23] For Indigenous Australians living in remote areas, cancer deaths are 2.5 times higher than for non-Indigenous Australians living in non-remote areas.[23] Available data, although limited, show that Indigenous Australians are disproportionately affected by rare and less common cancers (including liver, oesophageal and head and neck cancers),[23] highlighting the urgent need for action in this area. A holistic, community-controlled approach with greater representation of Aboriginal and Torres Strait Islander peoples in the cancer care workforce is needed to address these disparities and improve cancer outcomes.

Similarly, people from lower socioeconomic groups and culturally and linguistically diverse (CALD) populations face significant barriers in cancer care, including financial constraints, health literacy, and language barriers.[24, 25] This correlates to lower participation in clinical trials and worse health outcomes. Targeted interventions and tailored support for people from lower socioeconomic and CALD populations are also crucial to ensure equitable access to cancer prevention, diagnosis, support and treatment services.[26]

JOSÉ'S STORY

Força com Deus. God gives his toughest battles to his strongest soldiers.

My father, José, was diagnosed with stage 4 glioblastoma, and our journey through his treatment highlighted the significant challenges posed by language barriers in healthcare.

Navigating healthcare when English is not your first language is a constant battle. As a first-generation European family with no real roots or support, we learned early on that we had to navigate and assist our family within the "system". This experience brought us closer as a family unit, knowing we always had each other's backs.

The challenges began when my father collapsed at home in January 2024. The ambulance arrived and checked his vitals, assuring us he was okay. Despite their reassurances, my mother's fear persisted, so we insisted he be taken to the hospital. Only then did they perform an MRI, which had to be done at Concord Hospital because the machine at Canterbury was broken.

When symptoms first appeared, all staff in the emergency room and hospital were hesitant to provide any medication, despite my father's distress and pain. It took two to three days of persistent pleading before he received any relief in the form of painkillers and anti-anxiety medication. I had to confront the staff and push them to act. It was heartbreaking to realise that our limited English likely contributed to these delays. If my father had been more fluent in English, his challenge would have come sooner, and we believe he would have received medication more promptly.

Initial symptoms appeared on a Saturday, and we received no updates as no one was available to review the results. This added to our stress and anxiety. However, once he was transferred to neurology at Concord Hospital, overall the staff treated my father well. Before this, he was in cardiology, we felt he was brushed aside. The staff kept saying it was a brain-related issue, and we had to wait for a bed in neurology. Only when my brother and I kept probing for updates did they finally act.

My father did not feel comfortable being alone, so we had to have someone with him around the clock for support. The doctors did not properly explain things to him, and he often could not interpret what was being said. This meant my mother had to stay with him every night, which the nurses kindly allowed.

Navigating his radio and chemotherapy treatments was also a challenge, especially when they showed imagery and explained things. Only through our own research were my brother and I able to understand the communication in normal terms. We felt the doctors tried to play down the severity to avoid worrying us. We took meticulous notes during pre-operation meetings and showed a strong presence, which helped. Initially, they pressured my father to sign off on surgery, but we asked for more time to make an informed decision.

This experience has revealed the painful reality that language barriers can significantly impact the quality of care received. We learned to advocate fiercely for our family, to be their voice, but it shouldn't be this difficult. No one should feel powerless in such critical moments. Despite the challenges, we remained united, knowing that we had each other's support.

Financial toxicity

Any cancer diagnosis can be financially stressful, with reduced earning capacity and additional costs. However, for people with rare and less common cancers, high out-of-pocket costs can be crippling because:

- diagnosis is often protracted, with multiple GP and specialist appointments and out-of-pocket costs for diagnostic tests, which are often not government subsidised
- treatments are less accessible, with many paying for off-label medicines
- specialist expertise and treatments are centralised, requiring people to travel further, find accommodation near treatment centres and cover costs while away from home.

The financial strain of a rare cancer diagnosis typically increases over time due to mounting treatment, pharmacy, and travel costs, coupled with the impact on the family's earning capacity and ability to work. Many people experience significant financial strain including missed mortgage repayments and the inability to afford basic utilities or groceries.

Worry for the family's future can be overwhelming and lead to extremely difficult decisions. For example, does the family fund treatment that could give them more and better-quality time with loved ones using funds now, such as re-drawing on a mortgage or accessing superannuation early, or decide not to pursue treatments and leave the family with some financial stability for the future? Publicly sharing their private experience to crowdfund, often as a last resort, can also cause feelings of shame, embarrassment, and a loss of dignity.

RARE CANCERS AUSTRALIA

1 in 3 people

who call Rare Cancers Australia's navigator support service report financial stress because of their rare cancer diagnosis in their first call. Most people report financial stress and hardship at some stage in their support journey.

> "Today's cost of living crisis is already an overwhelming challenge for many families. Add a rare or less common cancer diagnosis on top of this and it becomes debilitating. I have supported many patients who have resorted to drastic measures in order to meet this financial burden. But this double-edged sword means less financial stability for the family's future."
>
> **Jaime Macedo, Cancer Navigation Program Lead, Rare Cancers Australia**

“The current healthcare system discriminates against rare cancer patients. We easily slip through the cracks as we are often not candidates for clinical trials, funded genetic tests and treatments, or Pharmaceutical Benefits Scheme (PBS) listed drugs which could prolong life.

I have had to access life insurance to afford emerging new treatments at $12,000 a month, and the next best treatment for me will cost $175,000 a year! A sound investment given that these treatments enable me to witness many family milestones. But why should I be penalised for having a rare or less common cancer? It should not be a competition between cancer types.

If you became one of the one in two Australians diagnosed with cancer, would you want to be the patient that slips through the cracks? Would you know where to look for the best treatments, which may be overseas, or be able to pay hundreds of thousands of dollars for them? As someone who has worked hard and paid taxes your entire adult life, wouldn’t you expect the government to fund the best standard of care available?

Any cancer is financially toxic – but to be further financially penalised due to a rare pathology seems unethical.”

Caitlin, diagnosed with stage 4 clear cell ovarian cancer in 2017

Psychosocial impact

People with rare and less common cancers can experience significant psychological distress, including anxiety, depression, fear, isolation, stigma, and guilt.

There is increasing recognition of the psychological impact of any cancer diagnosis, with the peak of psychological distresses, such as depression and anxiety, at the time of diagnosis and the effect continuing throughout treatment.[27] For people with rare and less common cancers, this can be intensified by:

- lack of information and support for their specific cancer type
- lack of evidence and clinical trial data to support decision-making
- poor or unknown prognosis with limited treatment options
- financial stress
- isolation due to lack of peer support.

Many people report feeling isolated from the broader cancer community, with a sense of being forgotten or left behind compared to those with more common cancers.

Studies have found significantly higher rates of psychological distress, anxiety and depression among rare cancer patients compared to the general population.[28] This can hinder treatment and recovery, as well as quality of life and wellbeing. Access to appropriate psycho-oncology and mental health support is critical, but often lacking.

> "When I was diagnosed, we didn't know what to do, who to contact, who to ask for help, or where to look for research. It's like this awful diagnosis was just dropped in our lap and we were left to figure it out. It all happened so quickly, but with such limited information. We felt very overwhelmed and a bit lost."
>
> **Noel, diagnosed with stage 4b thymic carcinoma in 2022**

Challenges accessing specialised care and expertise

Most cancer treatment centres and experts are based in metropolitan areas, requiring people to travel and stay away from home to access specialised treatments. This carries a considerable psychosocial and financial burden, with extended time away from jobs, support systems, and families. For some people, the practicalities of accessing specialised care are too significant, so they decline treatment options.

Treatment can last many months and often patients and their families need to relocate for its duration. For example, someone undergoing a stem cell transplant must stay within 30 minutes of the treating centre for three months. This compounds the financial pressure, with costs of accommodation, meals, and other medical expenses. It also means families are split apart or must give up or relocate work and school to stay together. This can be particularly distressing for those who live remotely. While patient assisted travel schemes are available in each state and territory, they can be inadequate in covering out-of-pocket costs.[29, 30] Significant variations between state and territory government schemes, insufficient subsidy amounts, and the exclusion of patients participating in clinical trials from most schemes create additional burdens.

Aboriginal and Torres Strait Islander peoples face additional barriers to accessing specialised cancer care, including the lack of culturally safe services, the limited availability of Indigenous health workers, and the need to travel away from Country and community for treatment. Embedding cultural safety and community partnerships into cancer services is vital to improve access and outcomes for Indigenous Australians.

Limited clinical trials and research

Clinical trials are essential for providing treatment options for patients with rare and less common cancers, especially when standard treatments are exhausted or unavailable. However, there are significantly fewer clinical trials for rare and less common cancers compared to more common types. This disparity is due to difficulties in recruiting sufficient patient numbers and lower financial viability for research sites and sponsors.

It is estimated that only 8% of cancer patients in Australia participate in clinical trials.[6] Barriers to participation include:

- a fragmented health system, with varying trial protocols and trial sites across jurisdictions
- difficulty navigating clinical trial databases and limited patient and clinician awareness of available trials
- high level of health literacy and clinician time required to navigate relevant trials
- availability of trials in Australia and exclusion criteria that prohibit participation
- limited knowledge of available trials among clinicians, including general practitioners.

Small patient numbers result in inadequate data for reimbursement. This can reduce the commercial incentive to develop treatments for rare cancers. The design of clinical trials is a significant issue; pan-tumour basket trials, which include people based on specific genetic mutations or biomarkers rather than location of the cancer, offer a promising solution by pooling data across different cancers.

Occasionally people with rare cancers and their families must consider travelling overseas to access clinical trials that are not readily available in Australia. However, the Medical Treatment Overseas Program[31] does not provide for access to clinical trials, adding to the financial burden and stress faced by people with rare and less common cancers for whom there are no options at home.

RARE CANCERS AUSTRALIA

Rare Cancers Australia has facilitated access to clinical trials for one in four of the people we support. Over one-third have been referred to Molecular Screening and Therapeutics study Precision Oncology Screening Platform Enabling Clinical Trials (MoST or PrOSPeCT) for access to free genomic testing and clinical advice on matched therapies.

CASE STUDY

Interview with Dr Michelle Harris, gynaecological oncologist, Darwin

Gynaecological oncologist, Dr Michelle Harris, didn't expect to end up practicing in the Northern Territory (NT). She happened to be up there visiting friends when the pandemic began and she was called upon to manage the hospital's gynaecological oncology service, as they were no longer going to be able to fly surgeons and patients in and out.

"That's how Australia's newest gynaecological cancer centre was developed, purely as a Covid emergency response. I quickly learnt how important this centre was to the Territory, in particular for the indigenous women living in remote areas, so I decided to stay on after the pandemic to ensure the service continued," Michelle reflects.

Four years later, Michelle now sees 15-20 patients every week, many of whom are Indigenous women. Her office is filled with beautiful local Aboriginal art, all gifts from her patients.

"I work really hard to establish trust and consistency with my patients, and it is the main reason I have stayed on here, so that trust is not broken. Things aren't done quickly here. Trust takes time and unfortunately for a person with a rare cancer diagnosis, and the bulk of gynaecological cancers are rare, time is something they don't have much of.

"Even the time and effort it takes for a patient to make their way to Darwin for a consultation or treatment can take hours, even days, of travel. In other States, each of the major metropolitan cancer centres have been set up so that they cover a part of the regional geography of that jurisdiction. But in many ways, the NT was left off that map.

"Whilst the population isn't big here, the level of illness is and the level of difficulty of managing that illness is extraordinary, mainly due to the distance. I have people travelling more than 2,000 kilometres to see me from some of the Central Australian communities. It's a 14-hour bus ride, one way, for the patients from Tennant Creek. For others it's a bus or two, an overnight stay in Alice Springs or Gove or Katherine, then a flight. Sometimes a patient has three children at home and a husband on dialysis and we've got to somehow work around those logistics to provide that person with the same level of care."

When asked how she thinks this added burden of travel and limited accessibility makes her patients feel, Michelle pauses for a while.

"I think they feel isolation and confusion," she says. "But there's a sort of pragmatic acceptance that is displayed, which is quite admirable. There is so much disruption to their lives just to simply get to the appointment, that you can bet they will be there when the day comes. Whereas, when I was working in Sydney, we'd have cancellations regularly because a patient's daughter was graduating from pre-school or the like. The pragmatism is quite different.

"Then there's the other patients I see who simply choose to not go ahead with their treatment, and this can be for a number of reasons, not just because of the travel. =

"Language and culture, for instance, are significant barriers, particularly for the First Nation's women I treat. Whilst we have a remarkable interpreting system up here covering about 100 different languages, there is still a disconnect between myself and the patient as a result. Some anatomical and treatment concepts are not translatable, so important information can get lost.

"Decision making itself is also often different with First Nations women. Sometimes they're not the only decision maker of whether they proceed with treatment or not – sometimes it will be the aunties, mother or grandmother in the family, sometimes it's the men in the family.

"It can feel frustrating and slow, but I have learned that time is important. Without allowing for it, trust can be lost and then that has an impact not just on that case, but on other cases from that community."

When asked about what could be done to address the challenges so many of her patients experience, Michelle candidly replied:

"Unfortunately, I haven't got a lot of solutions, there are a lot of problems. I hope that maybe in a few more years, together with NT women and health executive support, we'll be able to turn that around.

I didn't come with any skills in how to set up a service essentially from scratch – I trained very specifically as a gynecological cancer surgeon. We don't have the usual infrastructure of a cancer service in the NT. I'm recruiting a senior registrar and hope to get a clinical nurse specialist and social worker or psychologist too. It's a very different way of working here than in the big centres. "One practice-changing resource that I utilise often has been the Australian Rare Cancer (ARC) Portal, which provides me with access to detailed genetic and molecular analysis and offers expert guidance on treatment options for rare cancer patients. "The ARC Portal keeps me updated and connected as a clinician working in an isolated setting. It is a lifeline in providing high level cancer care in a regional setting where there are many geographical, logistic and financial challenges.

"It can feel frustrating and slow, but I have learned that time is important. Without allowing for it, trust can be lost and then that has an impact not just on that case, but on other cases from that community."

"It provides an extraordinary service for not only clinicians, but also patients, bridging an equity gap for our regional and First Nations patients by providing them with access, information and new treatment options.

When asked what inspires her and brings hope for the future, Michelle lights up and replies:

"Oh that's really easy…it's the patients and the families. I've got amazing patients, and I have some small skill that can help them to live. One of my mentors, Professor Neville Hacker, once said to me, "Michelle, your patients will teach you more than I will." He certainly taught me an enormous amount, but the women I care for show me their world, their community, their culture. From these women I learn about grace, trust, when to be fierce, and when to be accepting. I have a unique job in a unique place, and I know it is a position of great privilege," she says as she smiles and looks at one of her Aboriginal art paintings on her office wall.

LUNA'S JOURNEY

Shared by Samantha, Luna's mum.

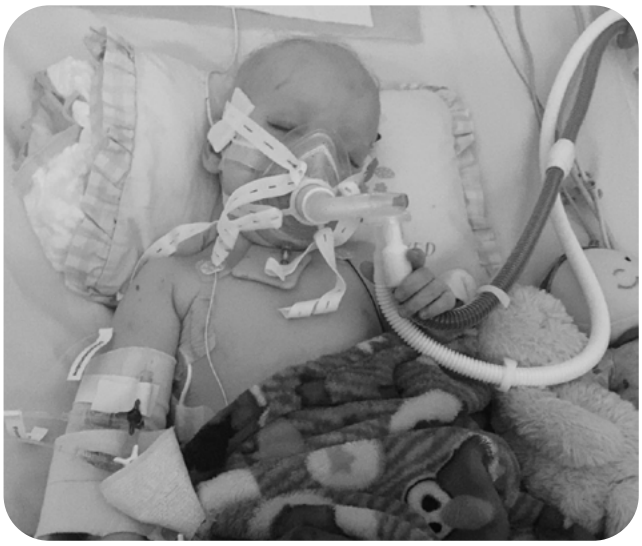
Luna is a bright, bubbly, adventurous two-year-old. It is her luminous smile, incredible strength and bravery that keep us going through the toughest time of our lives.

In July 2023, we heard the six little words no parents ever want to hear: **'I'm sorry, your child has cancer'.** She has high-risk stage 4 neuroblastoma – a rare and aggressive childhood cancer. Only 40 children per year in Australia are diagnosed with neuroblastoma. The five-year survival rate is 50%.

It took 22 months and too many hospital admissions, GP visits, specialist visits, misdiagnoses and trips to the emergency department to reach the correct diagnosis. Just two weeks prior to Luna's neuroblastoma diagnosis, we were told "kids get sick, deal with it" by one GP. We felt very disheartened and shocked as this doctor did not even examine Luna.

We always knew something was not right. However, never in our wildest dreams would we have imagined something so insidious. Luna's little body was riddled with cancer.

Within days of diagnosis, Luna was thrown straight into an intense treatment plan: five rounds of induction chemotherapy, surgery, high-dose chemotherapy, two bone marrow transplants, multiple rounds of radiation and immunotherapy. Luna has had numerous blood transfusions, platelet transfusions, biopsies, and central lines embedded in her chest for chemotherapy and other intravenous medicines. She has suffered horribly from serious side effects from the harsh treatments.



During her high-dose chemotherapy and bone marrow transplant (BMT) earlier this year, Luna was transferred to the Paediatric Intensive Care Unit (PICU) for two varying degrees of respiratory failure – she had had a major pulmonary haemorrhage (bleeding from the lung). There they diagnosed her with transplant associated thrombotic microangiopathy (TA-TMA), pulmonary veno-occlusive disease (P-VOD) and immune thrombocytopenic purpura (ITP). We're advised the odds were stacked against her and there was an incredibly high chance she would not survive. Luna fought bravely for two weeks in PICU and astonished all of her medical teams with a speedy recovery. It was anything short of a miracle. To this day, Luna is still suffering from the harsh effects of BMT, requiring weekly injections to help manage her ITP.

Luna's spirit is amazing. She lights up the hospital and finds joy wherever she can, like the way she gets excited about sleepovers at the 'nurses' house' (hospital). She finds her ways to cope. From the get-go, Luna has had a nasogastric feeding tube (NGT) to help improve her nutrition and easily administer daily medications. Replacing this tube is extremely traumatic for Luna and for us. We must forcefully hold her down, while she is screaming, crying and begging us to let her go. As her parents, we want to do everything we can to protect her and shield her from harm. It is a necessary evil.

On a journey like Luna's, establishing a strong, trusting relationship with your medical team is essential. This is the case with her lead oncologist—who is, quite literally, a walking, talking angel. Countless times during Luna's BMT, her oncologist stood by our side, supporting us when we felt unheard and backed into a corner. But we haven't always had all the information we need, and other medical professionals have dismissed our concerns and our questions. I understand I am not a medical professional, but as a mother, I know my child best. Trusting your instincts and advocating persistently for your child are crucial until you are satisfied with the care provided.

Caring for a child with a life-threatening illness brings numerous additional costs. I am Luna's full-time caregiver. I had to take unpaid leave from my job, and my fiance often needs to take unexpected time off work, and has taken a leave of absence from his tertiary studies. These hurdles

have significantly impacted our career paths, prospects and future opportunities. We are both incredibly humbled and grateful for our workplaces being flexible with our new life.

We incur a lot of extra expenses, including daily prescription medications, specialised formula, supplies and equipment, hospital stays, doctor visits, and the costs of being away from home. Luna will need ongoing support like occupational therapy and physiotherapy post-treatment, so we need to save for that. We have also sought out holistic therapies to undo, complement and resolve many of the toxicities Luna has been exposed to, which our insurance does not cover.

One of our biggest worries has been trying to save enough money to take Luna overseas to access difluoromethylornithine (DFMO). Her oncology team is throwing everything at this horrible disease. DFMO is available in the US and research has proven it can reduce the chances of relapse by 25-50%, improving the overall survival rates from 50% to 75%.

We were trying to raise $500,000, which was very daunting and exhausting for our family. We were most anxious about having to take a critically ill and severely immune-compromised toddler overseas. That's why we campaigned alongside other families in the same situation to get DFMO here in Australia.

We cannot begin to describe our relief and joy when the Federal Government recently announced that it would fund DFMO for children like Luna. A huge weight was lifted from our shoulders.

We want to see a world where people with rare cancers, like paediatric cancers, can access the latest, most promising treatments and receive world-class care here at home.

We share Luna's story, our family's story, in the hope that it can help others and pave the way for faster access to kinder treatments and more hope for people with rare cancers. There needs to be more awareness. Before our daughter was diagnosed with stage 4 neuroblastoma, I had no idea there were such aggressive, fast-growing and insidious cancers such as neuroblastoma. Rare cancers bring their own set of hurdles. Patients and families are often isolated with not much information, rare cancers are expensive, and often leave families seeking medical treatments overseas.

We now live by the mantra: you are your child's biggest advocate. If you can't advocate and speak up for them, who will? We will never stop raising awareness.

Sometimes real-life heroes live inside little children fighting big battles! Luna is our real-life hero.

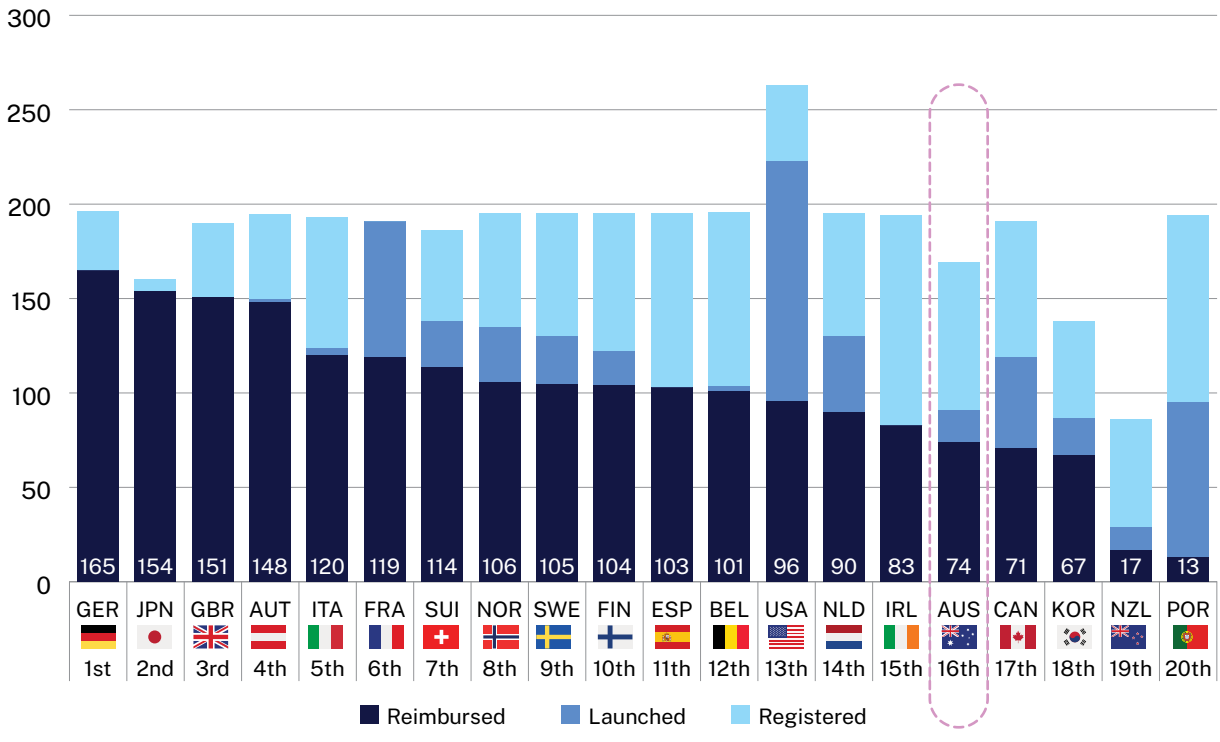
We now live by the mantra: you are your child's biggest advocate. If you can't advocate and speak up for them, who will? We will never stop raising awareness.

Delayed and denied access to medicines

On average, Australians wait 442 days for PBS reimbursement of a new cancer drug following approval by the Therapeutic Goods Administration (TGA).[32] For people with rare cancers, the wait is even longer.

> "Australia is highly regarded as a destination for clinical trials, but our reimbursement environment is seen as challenging by the global innovative pharmaceutical industry. Companies seeking to make innovative medicines available to Australians face system inefficiencies, a narrow recognition of the value of medicines, and opaque decision-making frameworks that ultimately result in delayed access to affordable medicines for patients. Various reviews have identified that Australia's system is not fit for purpose, has not kept pace with advances in science and technology, and patients are left waiting for new treatments that are available elsewhere in the world. We know what needs to change – now it is time to get on with it."
>
> **Elizabeth de Somer, Chief Executive, Medicines Australia**

Figure 4: Number of reimbursed new molecular entities registered, launched, and reimbursed per country in 2016-2021 (Adapted from *Medicines Matter 2022*, Medicines Australia)[32]



Medicines Australia reviewed 472 new molecular entities (NMEs) that were first registered in at least one of the 20 Organisation for Economic Co-operation and Development (OECD) countries from 2016 to 2021.[32] They recorded which were registered, launched, and reimbursed by each country. Australia ranked 16th of 20 OECD countries *(see Figure 5)*.[32]

The TGA evaluates the safety, quality, and efficacy of drugs, while the Pharmaceutical Benefits Advisory Committee (PBAC) assesses drugs for cost-effectiveness and provides subsidies via the PBS. Typically, TGA and PBAC evaluations occur on an indication-by-indication basis, although there have recently been submissions for multiple indications.[33] However, at the time of publication, no multiple indication submissions have been approved in Australia.

The paucity of clinical trial data for rare cancers further compounds this issue. Rightly, Australia has a very high threshold for evidence, however, for small patient populations that evidentiary threshold may never be met. Pharmaceutical companies may be deterred from seeking PBS listing in Australia due to the limited evidence, high rejection risk, and resources needed to prepare a submission for a relatively small patient population. As a result, people with rare and less common cancers in Australia continue to miss out on the best available therapies.

Once a drug has been approved for its initial indication, doctors can prescribe it for other conditions if they deem it clinically appropriate—this is known as 'off-label use'. However, such use is not subsidised and often incurs high costs for the patient, making it inaccessible for many. Additionally, medical oncologists report feeling uncomfortable discussing unfunded treatment options with their patients. They are often conflicted about presenting these options, particularly when they might cause financial strain.[34] Accessing therapies in this manner also misses the opportunity to generate clinical data and further contribute to the evidence for approval, as would be the case if the person accessed it through a clinical trial.

"There is a substantial inequity in the Australian health system for people with rare cancers. In February this year, I was diagnosed with stage 4 epithelioid sarcoma. The conventional treatment offers very little, with a one-year survival rate of less than 10%.

I decided, with my oncologist, to try immunotherapy, which has had good results with some other cancers. The particular drug is PBS approved for 11 other cancer types (and approved for 39 cancer types in the US). However, it is not subsidised for my rare sub-type. I was quoted a cost of $62,000 for a six-month treatment course.

My response to this previously untried treatment for this tumour has been extraordinary. All cancer symptoms settled over two weeks and my PET scan at eight weeks showed a substantial reduction of tumour mass and even elimination of cancer in some parts of my body. My oncologist expects further reduction in tumour mass, if not elimination of tumour tissue, and describes my situation as a 'magic moment'.

Our health system in Australia subsidises expensive cancer drugs when there is trial evidence that there is a reasonable chance of a beneficial response. The inequity is that people diagnosed with rare cancers do not receive such support because trials have not been, and cannot be, performed. An enormous and unanticipated financial burden on families in this situation is deplorable."

Richard, diagnosed with stage 4 epithelioid sarcoma in 2024

A Rare Cancer Moonshot

There is no single action that will deliver equity of outcomes and experience for people with rare and less common cancers. It requires strategic investment, policy reform, and collective action across six priority areas, working within – and pushing further on – the strategic objectives of the Australian Cancer Plan and respective state cancer plans.

A Rare Cancer Moonshot demands a concerted effort across government, healthcare, research, industry, and the community to radically accelerate progress so that by 2035:

- more than 90% of people diagnosed with cancer survive at least five years after diagnosis, regardless of rarity
- there is true equity of experience for everyone diagnosed with cancer.

Early detection and timely diagnosis

Early detection and timely and accurate diagnosis are crucial for improving outcomes in rare cancers. Cancer survival increases significantly if detected and treated at an early stage.[35]

However, rare and less common cancers present unique diagnostic challenges. These factors contribute to later presentation, poorer survival rates, fewer treatment options, and higher costs for both health systems and individuals.[6]

Many rare and less common cancers lack distinct 'red flag' symptoms. Common ailments such as fatigue, weight loss or bloating can have a range of non-cancer-related causes. Other more common conditions are investigated first which can mean numerous tests and appointments before a suspected cancer referral. Low symptom awareness among the public and clinicians contributes to delays in seeking help and making referrals, and there is widespread variation in the information, professional training and diagnostic tests available to GPs in different parts of the country.

To transform survival outcomes for people with rare and less common cancers, we need a greater focus on finding new early detection methods and accelerating complex diagnostic pathways. Promising new developments include liquid biopsies, which analyse circulating tumour DNA in a non-invasive blood sample,[36] with testing underway in Australia and abroad. Advanced imaging techniques, including functional and molecular imaging, are enabling more precise characterisation of rare tumour types.[37] Artificial intelligence is also showing potential to detect early-stage pancreatic and other cancers through blood and imaging biomarkers.[38, 39] Australia needs to push ahead with research in this space, to find and capitalise on the diagnostic tools of the future.

Awareness, early diagnosis and screening were key focus areas of the recent Senate inquiry into *Equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer.* The Committee made eight recommendations to urgently remove and reduce existing barriers to swift and accurate diagnosis of rare and less common cancers,[6] which should be accepted and adopted.

> "The symptoms of neuroendocrine cancer are so vague: diarrhoea, bloating, flushing, wheezing –it could be anything. Because the diagnosis is delayed by an average of five to seven years, symptoms are often protracted. This is also the case for other rare and less common cancers. 60% of these patients have stage 4 disease, which means it's spread to other organs, like the liver, and the chance of cure is often lost."
>
> **Meredith Cummins, Chief Executive Officer, NeuroEndocrine Cancer Australia**

Screening and risk-based surveillance

While Australia has well-established screening programs for breast, bowel and cervical cancers and a soon-to-be launched national lung cancer screening program, there are no national screening programs for rare and less common cancers.[40] Population level screening approaches will likely never be appropriate for these cancers, given the small number of people diagnosed and the lack of accurate and affordable tests that would allow large-scale testing.

However, risk-based and targeted approaches to screening and surveillance offer hope for earlier detection and identification of cancer in asymptomatic patients. As our knowledge of cancer aetiology deepens, we can better target awareness and develop screening and surveillance programs for at-risk groups. For example, monitoring people with liver cirrhosis, hepatitis B or hepatitis C, who have a 20 to 100 times increased risk of developing primary liver cancer, is an effective way to diagnose liver cancer earlier for people in those at-risk populations.[41]

Familial genetic risk is another way to stratify and monitor populations, such as increased testing and support for people with Lynch syndrome, which increases a person's risk of developing certain cancers including endometrial and colorectal cancers.[42] As precision medicine evolves, it will open further opportunities for prevention and early detection, leveraging individual genomic, environmental, and lifestyle data to identify people at risk and support them to take preventative steps and clinically recommended monitoring.

RECOMMENDATION

Deliver the Senate Inquiry recommendations:

The Australian Government should accept the 41 recommendations of the Senate inquiry into *Equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer.* There needs to be a nationally coordinated approach with states, territories, funded bodies, and the rare cancer community to implement the recommendations in full.

1

EMMA'S STORY

I was diagnosed with non-Hodgkin B-cell lymphoma in 2020. It isn't a rare cancer itself, but the way it presented was rare, as it appeared overnight and it typically presents in older people. I also have another condition which is very rare, lymphomatoid papulosis (LyP). There is debate as to whether LyP is itself a type of cancer but there is an understanding that it can cause lymphoma and other cancers, which is what happened in my case.

I live with my husband and two beautiful daughters in Sydney, which is where I grew up. My husband was born in Que Que (now Zimbabwe) and we met in Sydney while he was travelling on a holiday visa after completing a round-the-world trip on his motorbike. We've been married for 20 years this year. He is the most patient man in the world and I'm so grateful for the love he and my daughters have shown me during this stressful time.

I first noticed the LyP papules in 2017. They're small, raised areas that look like mosquito bites and then go away after six to eight weeks. I saw numerous dermatologists and other specialists and had lots of tests and biopsies done. No one was able to confirm what they were. I was given medications to try, creams to put on and told to stop scratching as I was probably causing the papules to spread! LyP is extremely itchy and so this made me feel guilty and that I was doing something to cause them.

In Jan 2020 (yep, just before Covid hit) I woke up one morning to a large lump the size of a golf ball in my right groin. I swear it was not there the night before! I went to my GP, who sent me to hospital, scans and a biopsy was done and it was confirmed as lymphoma. The doctor who treated me that day at Hornsby Hospital Emergency Department noticed the papules and asked me what they were. I could see his concerned look and wish I could go back to him and tell him he saw something that no one else did.

Tests confirmed I had stage 1 small lymphocytic lymphoma (SLL), which is a type of non-Hodgkins lymphoma. I saw five haematologists and each had a different opinion. I was so scared (and financially drained) and had no idea what to do.

I had none of the traditional symptoms of weight loss, night sweats etc. If someone had known what the papules were, I may have been more aware of the possibility of lymphoma. One haematologist I saw noticed the LyP and encouraged me to see a specialist dermatologist. I am so thankful I took his advice (even though it took me a few months before I made an appointment to see her). She has been fantastic and remains a crucial part of my care team. But she is one of very few specialists in this field and I am fearful of what will happen if she ever decides to retire.

Thankfully, the lymphoma had not spread beyond the lump in my groin, so my treatment was four weeks of daily radiotherapy. This was in the depths of the pandemic, so it was really isolating. We used to joke about it being a 'zap and go' service – I had to attend on my own, enter through a secret entrance way, and didn't see anyone else. It was all done so quickly. But it was so exhausting. The radiation kills the healthy cells as well as the cancerous ones so my body was working really hard to regenerate and heal. I struggled mentally, mainly from the feeling of isolation, the exhaustion and being unable to work.

I don't want other people to go through what I did. These cancers may be rare but if we had better knowledge about them then I bet there is more out there than we know!

Before my LyP was diagnosed, I was an emotional wreck. I felt like the medical profession just didn't care. I was on the receiving end of much complacency and judgement as I continued to try and find an answer about the condition I was suffering from, and the dermatologists I saw did not have any awareness of it as a rare condition let alone it being a pre-condition for lymphoma.

The last dermatologist I saw actually said to me, "I don't know what else you expect me to do". I gave up seeking answers after that comment. I learned to advocate for myself and research, research, research. I learned that some of the problems we think are big are not really that big.

In some ways, I feel lucky that this happened during the pandemic. Our usual expenses were lower, which helped as we needed every last cent to cover the medical costs. I was not working, my husband needed to take time off to support me and our girls (who were being homeschooled because of the pandemic). Our out-of-pocket expenses piled up for tests and specialist appointments. We had to move out of our family home (as we lived 1.5 hours from Sydney city in a rural area) and rent a property that was closer to the hospital. We almost lost our house in the process as it took time for it to be rented out.

Now, I am so thankful to be able to go to work each day as it means that I am well. After having 18 months off that were out of my control, I don't want to miss another day! I feel more informed and empowered now and have been bringing together a medical team that I am more comfortable with. My GP, who I found 18 months ago, sees me as a whole person and is always advocating for me. She takes a holistic approach and provides gold star support and care. We need more professionals like her.

LyP is an incurable disease and it could cause another cancer. I have six-monthly monitoring and so far there is no sign of recurrence. It is scary – no one knew the first time around, so how will they know next time?

I don't want other people to go through what I did. These cancers may be rare but if we had better knowledge about them then I bet there is more out there than we know! To this day, I do not know of another person with LyP – but I do know they are out there somewhere. Let's pull together what we do know and see where we can take it.

Don't give up, keep asking questions and do your research. Ask for help (like through RCA) as you are not alone.

Affordable access to comprehensive personalised medicine

Genomic testing plays a critical role in guiding treatment planning for people with rare and less common cancers. By identifying the specific genetic variations driving a person's cancer, precision medicine approaches can match people with therapies tailored to their unique tumour profile.

This targeted strategy has the potential to dramatically improve outcomes. A retrospective analysis of data from MoST shows that 37.5% of patients carry biomarkers with good clinical evidence for therapeutic benefit.[43] In the first 3,383 patients enrolled onto MoST, patients who accessed matched therapies had a median survival double that of patients receiving unmatched therapy.[44]

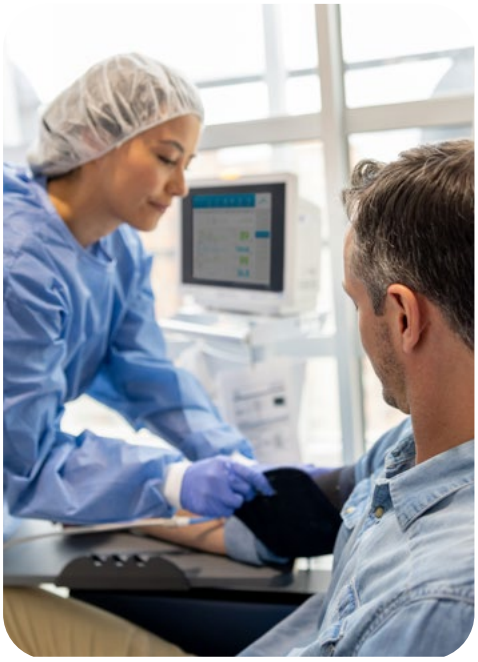
Despite the immense potential of precision oncology, access to genomic testing remains a significant challenge for many people in Australia. The high cost of tests can put them out of reach for most people. Lack of reimbursement and limited testing infrastructure, particularly outside major cancer centres, further compound the access barriers.

Government funding for genomic research and translational studies is advancing research and access to genetic testing for people living with rare cancers in Australia. However, there remains a serious challenge in moving research into mainstream clinical practice. The long-term sustainability and scalability of projects like Zero Childhood Cancers and PrOSPeCT require dedicated, ongoing funding and coordination.

Australia urgently needs a nationally coordinated genomics strategy, to rapidly increase genomic testing capacity, reporting infrastructure, and equitable access across all states and territories. This strategy should include a robust reimbursement framework for genomic tests, targeted investments in expanding testing facilities and workforce, and clear referral pathways for timely testing at diagnosis.

Precision Oncology Screening Platform Enabling Clinical Trials (PrOSPeCT)

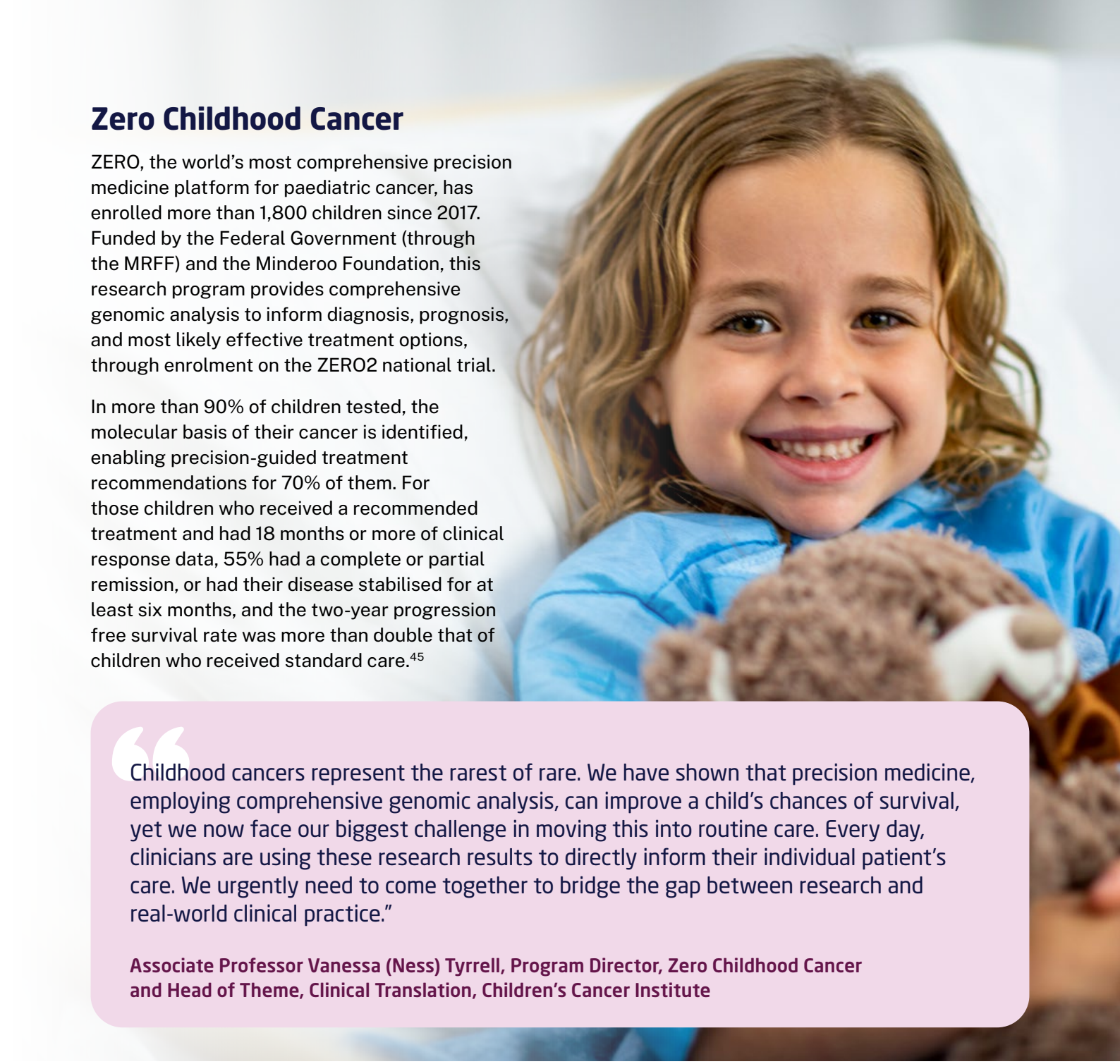
PrOSPeCT provides research-funded genomic profiling to patients in Australia with advanced and incurable cancers and matches patients with recommended targeted treatments and clinical trials, building on the results of the MoST study. There is a strong focus on increasing availability of genomic profiling for people living in rural, regional, and remote areas, with 38% of enrolled patients to-date from rural and remote areas. More than 5,000 patients have been referred to PrOSPeCT by over 750 clinicians since it opened in January 2024, with a target of 23,000 patients within the first 27 months.[44]



Zero Childhood Cancer

ZERO, the world's most comprehensive precision medicine platform for paediatric cancer, has enrolled more than 1,800 children since 2017. Funded by the Federal Government (through the MRFF) and the Minderoo Foundation, this research program provides comprehensive genomic analysis to inform diagnosis, prognosis, and most likely effective treatment options, through enrolment on the ZERO2 national trial.

In more than 90% of children tested, the molecular basis of their cancer is identified, enabling precision-guided treatment recommendations for 70% of them. For those children who received a recommended treatment and had 18 months or more of clinical response data, 55% had a complete or partial remission, or had their disease stabilised for at least six months, and the two-year progression free survival rate was more than double that of children who received standard care.[45]



> "Childhood cancers represent the rarest of rare. We have shown that precision medicine, employing comprehensive genomic analysis, can improve a child's chances of survival, yet we now face our biggest challenge in moving this into routine care. Every day, clinicians are using these research results to directly inform their individual patient's care. We urgently need to come together to bridge the gap between research and real-world clinical practice."
>
> **Associate Professor Vanessa (Ness) Tyrrell, Program Director, Zero Childhood Cancer and Head of Theme, Clinical Translation, Children's Cancer Institute**

RECOMMENDATION

Develop and implement the National Genomics Strategy:

Genomics Australia (once established) and Cancer Australia should work with the Australian Government Department of Health and Aged Care, and State and Territory governments to deliver a National Genomics Strategy and National Framework for Genomics in Cancer Control that:

- delivers comprehensive and cost-effective genomic profiling as standard of care, starting with patients who have the highest unmet need
- equips and supports all health systems to introduce genomic profiling and matched therapies as a coordinated part of cancer pathways, including the smooth transition of comprehensive genomic and functional studies methods into clinical practice.

2

The benefits and challenges of precision medicine are explored in more detail in *Rarefication: Personalised medicine in the genomic revolution.*[21]

Equitable access to subsidised treatments and clinical trials

> There are some very exciting and hopeful treatments coming through investigation with personalised tumour neoantigen vaccines and immune system engaging antibodies, which really could move us to a new frontier in cancer care."
>
> **Dr Malinda Itchins, Medical Oncologist, Royal North Shore Hospital and North Shore Private Hospital**

In the absence of screening and early detection mechanisms, access to effective treatment is critical for people with rare and less common cancers. Over the past decade, breakthroughs in precision medicine and the development of innovative therapies have provided a beacon of hope.

Our evolving understanding of the genomic drivers and origins of cancer is paving the way for targeted, more effective, and less toxic treatments. The move from broad-spectrum cytotoxic drugs to targeted therapies and immunotherapies offers hope for many rare and less common cancers. Cell and gene therapies have recently been introduced into the Australian market, but it is taking too long to achieve subsidised access for people who need them. Unfortunately, despite significant breakthroughs in precision medicine and innovative therapies that transform cancer care, the PBS has not kept pace with the ability to assess and approve newer therapies and patients miss out on subsidised access as a result.

Despite the advocacy of Rare Cancers Australia and many others over the past decade, a growing number of people in Australia are unable to access innovative cancer technologies, therapies and services compared with other OECD countries. Australia is one of the five worst countries in terms of 'indication coverage' for cancer medicines – intended as the number of indications covered by the public healthcare system – that do achieve subsidised access.[46] Australia needs a modern and agile HTA process that better balances evidence-based decision making and cost-effectiveness with equity of access for people with rare cancers.

The promise of new therapies

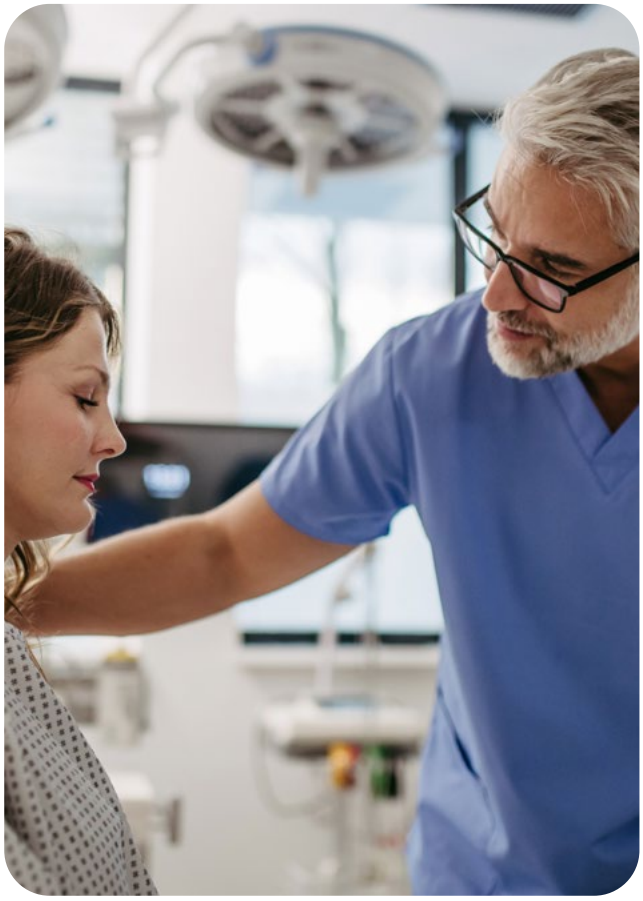
We have seen dramatic advances in cancer therapies over the past 50 years. From the identification of oncogenes and tumour suppressor genes to the development of targeted therapies, immunotherapies and nuclear medicine; there are many new and emerging therapies that are already here, or on the horizon. Ensuring that people have access to the best available therapies, at the earliest opportunity, requires effective horizon scanning. It is crucial that governments and healthcare systems anticipate and prepare for the adoption of new models of care. By proactively identifying and assessing the potential impact of upcoming therapies, Australia can develop the necessary infrastructure, train personnel, and establish patient support systems well in advance, to ensure a smoother integration of new treatments and minimise disruptions to patient care.

Australia has not been ready for these complex treatment modalities. With more on the way, there is an urgent need for healthcare systems to adapt and prepare for new models of care *(see Figure 5)*.

It is equally important that we make the most of existing best practice treatments. As Optimal Care Pathways are developed and implemented across more cancer types,[47] we need measures of whether patients are accessing the optimal care, and data to drive clinical reflection and best practice. For example, radiotherapy is being under-utilised, with 20% of patients not receiving it despite being clinically recommended for their pathway.[48] Contemporary radiotherapy methods including stereotactic body radiotherapy and hyper-fractionated radiotherapy could help to increase take-up, with shorter treatment rounds, lower costs for patients and fewer side effects through more precise targeting.

Tackling antimicrobial resistance is also vital in ensuring optimal use of existing and novel cancer treatments. Immunosuppression from cancer and its treatment makes people with cancer highly susceptible to infection. While better data is needed on infection prevalence and mortality, an 11-year study of patients at Peter MacCallum Cancer Centre showed 27% of inpatients with solid tumour neoplasm and 67% of those with haematological malignancy had at least one type of infection.[49] Antibiotic failure in patients with cancer increases the frequency of sepsis, sepsis-related mortality, and sepsis-associated costs of care.[50] We need a proactive strategy for optimising antibiotic use and encouraging new antibiotic development, to help protect people with cancer against infections and reduce infection-related mortality, morbidity and expense.

Barriers to accessing treatment are explored in *Counting the cost: The true value of investing in cancer treatment.*[5]



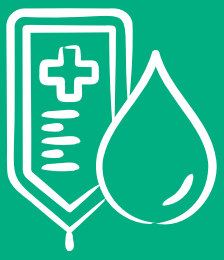
Horizon scanning

Cancer vaccines

stimulate the body's immune system to attack cancer cells. These vaccines can be preventative (such as the HPV vaccine) or therapeutic, aiming to treat existing cancers by targeting specific cancer antigens.

CAR-T therapy

involves taking T-cells from the body and engineering them to target cancer cells. These modified T-cells are then multiplied in the lab and reintroduced into the patient to attack the cancer.



Checkpoint inhibitors

are drugs that help the immune system recognise and attack cancer cells. They work by blocking proteins that prevent T-cells from attacking cancer cells, thus boosting the immune response against the tumour.

Combination therapies

use multiple cancer treatments with different modes of action at the same time. This approach can enhance effectiveness, reduce the likelihood of drug resistance, and improve patient outcomes by attacking cancer on multiple fronts.

Nuclear medicine

including radioligand therapy, uses a ligand (a molecule that binds to cancer cells) to detect and deliver a precise dose of radioactive isotope to kill the cells, minimising damage to surrounding tissue.

Oncolytic virus therapy

uses viruses that selectively infect and kill cancer cells, stimulating an immune response. While it is still in trials and not yet approved for widespread use, researchers are exploring various viruses for their potential to target different types of cancer effectively.

Targeted therapies

use drugs to precisely identify and attack cancer cells, usually by targeting specific molecules involved in cancer growth and progression.

Modern radiation therapy,

including stereotactic and hyper-fractionated radiotherapy, uses advanced technology to precisely target cancer cells while sparing healthy tissue. This method allows for higher doses of radiation to be delivered to the tumour with fewer side effects, improving treatment efficacy.

Figure 5: Paving the way for new treatments

System readiness components for advanced cancer therapies

Patient access and support

- Information to help patients understand their options, including side effects and practicalities of new treatments.
- Support for shared decision-making and informed consent.
- Update referral and treatment pathways including Optimal Care Pathways and clinical guidelines.
- Update patient reimbursement schemes to take account of practical requirements of new treatments.
- Expand or establish rehabilitation and support services for treatment and side effect management.
- Identify and address disparities in access to new treatments to ensure all patients can benefit.

Regulatory approval and compliance

- Clinical trials to establish safety, efficacy, and dosage; and to expand use to other cancer types.
- Therapeutic Goods Administration approval for treatment and companion diagnostics at same time.
- PBAC agreement and MBS schedule updates to enable subsidised patient access.
- Post-market data collection on effectiveness, side effects and long-term outcomes and social return on investment.
- Clarity of federal and state funding responsibilities and commissioning arrangements.

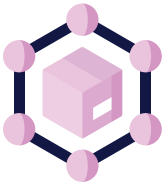
Infrastructure

- Identify delivery centres and ensure specialist equipment and patient suites are in place to administer therapies.
- Implement telehealth models where possible to increase access for rural and remote patients.
- Ensure appropriate data management in place, including integrated health record systems and ability to report patient outcomes and side effects.
- Universal availability of molecular screening, diagnostic imaging and other companion diagnostics.

Workforce and training

- Ensure tertiary education and training places available to meet future workforce requirements, including new specialties.
- Recruit and retain new roles required.
- Train existing healthcare teams to deliver new treatments and support patients.
- Network delivery centres with specialist expertise and advice on new treatments and patient suitability.

Supply chain



- Expand or set up labs for the production and quality control of treatments like CAR T-cell therapy and oncolytic viruses.
- Develop Australia's capacity and capability to manufacture therapies locally, where appropriate.
- Where manufacture remains offshore (e.g., radioisotope and CAR T-cell engineering), establish reliable and cost-effective supply for safe and timely transportation and storage.
- Ensure processes in place for biomaterial and hazardous material handling (including collection, transportation, and storage of patient samples and therapeutic agents).

CAR T-cell therapy: navigating uncharted territory

The introduction of innovative CAR T-cell therapy has revolutionised the treatment landscape for certain rare cancers, particularly relapsed or refractory large B-cell lymphoma. This groundbreaking personalised cancer treatment has shown remarkable efficacy, but its unique logistics and impact have posed unprecedented challenges for healthcare systems.[51]

For patients undergoing CAR T-cell therapy, the journey begins with the collection of their T-cells, which are then genetically modified to attack cancer cells. This process, known as leukapheresis, can be physically demanding and emotionally taxing. Once modified, the cells are infused back into the patient, where they proliferate and target the cancer.[52]

"When my haematologist told me he wanted to start me on CAR T-cell therapy straight away at the Peter MacCallum Cancer Centre my partner and I had just seen the removalists drive off with the contents of our unit for our new home in NSW.

Two months in Melbourne. Where would we stay? To our huge relief, Peter Mac and Rare Cancers Australia provided us with accommodation within walking distance of the hospital.

The treatment was tough – but successful. I am now in complete remission from my refractory mantle cell lymphoma. I cannot thank the amazing clinicians and staff at Peter Mac, and Rare Cancers Australia enough."

Richard, diagnosed with refractory mantle cell lymphoma in 2023

However, this treatment is not without its hurdles. The therapy requires patients to be near specialised centres capable of handling such complex procedures. This often means significant travel, extended stays away from home, and the logistical burden of coordinating with multiple healthcare providers. The impact of CAR T-cell therapy on patients can be profound. While many experience significant improvements, the journey is often marked by disruptions to their daily lives.

Rare Cancers Australia plays a crucial role in supporting patients through this process. From providing detailed information about the treatment to assisting with travel arrangements, RCA ensures that patients and their families are not navigating this journey alone.

My husband had to take time off work to care for me after I was discharged from the hospital. Living in the beautiful Blue Mountains and traveling to Westmead Hospital for treatment was very challenging for us. Without the ability to stay close to the hospital, we would have faced serious health safety concerns.

In the early stages, as an outpatient traveling long distances, the fuel vouchers significantly eased the financial burden, allowing my husband to focus on my care. Once I was discharged and needed to stay nearby for ongoing treatment, our beautiful accommodation in Parramatta was a godsend. It not only kept me close to the hospital but also provided a comfortable and serene environment to aid my recovery. Walking around the gardens each day, I could enjoy the fresh air and gradually build up my strength. The accommodation also allowed my sister to come and care for me, giving my husband a much-needed break. She had been anxious living interstate and unable to see me, so the support we received was deeply appreciated by her as well.

The grocery vouchers were incredibly helpful, especially since my husband Gary could easily walk to the nearby store to get fresh food for us. Gary has since returned to work, and I am gradually getting stronger each day, even though I am still immunocompromised, recovering, and often very tired.

There was a day when I felt particularly low after the flurry of doctor appointments and nurse visits had subsided, leaving me alone at home. On that day, a beautiful parcel arrived from Rare Cancers Australia, accompanied by an incredibly thoughtful note of support from Jaime. Once again, I felt deeply supported and immensely grateful, reminded that I am not alone on this unexpected journey."

Joy, diagnosed with diffuse large B-Cell lymphoma in 2023

Expanding access to clinical trials

Increasing participation in clinical trials is crucial for improving outcomes. Clinical trials provide patients with access to potentially life-saving treatments, further medical knowledge, and support development of new therapies. Importantly, people with cancer treated at centres that conduct clinical trials have better outcomes, even when not directly enrolled in a trial, due to the high standard of care and the innovative treatment options available.[32] Research environments foster learning, evaluation, and clinical excellence.

Improving the clinical trials landscape to support all people with cancer to participate in trials is imperative, as recognised in the Australian Cancer Plan.[53] Innovative trial designs and the use of telehealth can increase trial recruitment and reduce the burden of participation, which is particularly vital for improving outcomes for Australians living in rural and remote areas.

Australia has seen success in decentralised clinical trials, bringing trials closer to patients, especially in regional and remote areas. Programs like the Australian Teletrial Program, Queensland Cancer Clinical Network, and WA Country Health Services TeleTrial Program leverage technology to enable remote participation, reducing travel burdens and increasing diversity in trial populations.[54, 55]

Importantly, people with cancer in rural and remote locations had similar outcomes to those in metropolitan areas when enrolled in the same clinical trials.

Cross-country collaboration is key for impactful rare cancer research and trials, given the small numbers of patients. However, challenges remain in funding, infrastructure, and regulations for widespread adoption of teletrials and for trials taking place across different states and territories.

> “Clinical trials should be the standard of care. Especially when the standard of care fails our patients. Clinical trials should be a right of all patients”.
>
> **Professor David Thomas,**
> **Chief Science and Strategy Officer of Omico;**
> **Director, Centre for Molecular Oncology,**
> **University of New South Wales; and**
> **Head, Genomic Cancer Medicine Laboratory,**
> **Garvan Institute of Medical Research**

Health Technology Assessment reform and pan-tumour approvals

Australia's medicines approval system is currently under Government consideration through the HTA Review. Bold reform is needed to ensure faster, more equitable access for rare cancer patients and to significantly reduce the financial burden that many who must directly fund their care face. As the last four years have taught us, where there is an identified public health priority our HTA system is capable of rapidly assessing and approving diagnostics, vaccines and treatments for public use. By recognising the urgency of action required to address the burden of disease attributable to rare cancers and the need to act, we can make a difference to people living with rare and less common cancers now and into the future. This is a chance to set Australia up for the future, with a more responsive, adaptable, and sustainable mechanism for bringing new medicines, therapies, and associated tests to market.

We need a fundamental shift in how evidence is considered to better reflect the challenges posed by data limitations among small patient populations. This includes placing greater emphasis on, and recognising the value of, real-world evidence that can supplement limited clinical trial data and support regulatory decision-making. Involvement of people with lived experience is being explored through the current HTA Review, with lack of transparency and barriers to participation often cited as missed opportunities for communicating real world experience. Enhanced consumer engagement in regulatory processes aligns with best practice, and Australia should seek to optimise our approach. Data from novel trial designs, such as basket trials or umbrella trials, which consider multiple targeted therapies across multiple cancers, should also be considered, particularly where randomised controlled trial data are impossible to generate.

We also need to shift the way we think about the value treatments deliver for patients, their families and society more broadly. As we discussed in our 2022 report, *Counting the cost: The true value of investing in cancer treatment*, it is time for us to look beyond standard measures of cost-effectiveness based on clinical outcomes, and move to a model that appropriately assesses the social value that investment in cancer care delivers.[5] For every $1 invested in cancer treatments, $3.06 of social and economic value is created, showing the substantial long-term benefits of investing in new therapies.

Further, co-dependent technologies associated with the effective use of a medicine should be required to be assessed at the same time as the medicine or medical service it is used with, to prevent high costs for patients that could prevent access to an otherwise funded treatment.

> "Drug access is really important, but we are falling short before that point. Australia's slow, test-by-test approval of new molecular diagnostics means that doctors don't have the right tests available to practice precision medicine. We can't do the full suite of tests needed to work out which drugs are needed, so we are limiting the patient's options before we even get to the point of considering access to medicines and the challenges and costs there. We are way behind the rest of the world in getting the right suite of diagnostic tests done to enable precision medicine. We need bold reform."
>
> **Professor John Zalcberg, OAM, Consultant Medical Oncologist, Alfred Health and Professor (Emeritus), School of Public Health, Monash University**

The Government's recent announcement to fund DFMO (difluoromethylornithine) for children and young adults with high-risk neuroblastoma in Australia is a momentous step in making life-changing treatments available more quickly.[56] By funding the drug in parallel with formal HTA processes, the Government will immediately change the lives of Australian families who were otherwise fundraising to go overseas for the treatment, which offers the only option for some patients with neuroblastoma. Interim funding arrangements like this should be considered for other rare and less common cancers, allowing people with cancer to benefit from access to treatment sooner, and contribute to the real-world evidence base, while lengthy approvals processes are pursued.

As an immediate priority, the PBAC should consider how pan-tumour or 'tissue-agnostic' drug approvals can be listed on the PBS. This means approving a therapy based on a specific mutation or biomarker across multiple cancer types, rather than for each indication separately. This approach has been used in the United States since 2017, when pembrolizumab was approved by the FDA for people with tumours characterised as unresectable or metastatic, microsatellite instability-high or mismatch repair deficient (MSI-H/dMMR) solid tumours.[57] The FDA approval was based on data from 149 patients with MSI-H/dMMR cancers enrolled across multiple clinical trials; 90 patients had colorectal cancer and 59 patients were diagnosed with one of 14 other cancer types.[57]

By using a fast-tracked pathway to assess evidence to extend the use of a targeted medicine, more people with rare indications could benefit from earlier access to medicines and contribute to the evidence-base used to make funding decisions. While there may be concerns regarding the potential budget impact of expanding access to cover all indications for such therapies, treatment will only continue in patients while it is delivering benefit and therefore there is no wasteful cost, or loss of precious time for patients. The government simply 'pays for performance' – where the treatment works and a patient is showing benefits, they will continue to use it and the cost represents value.

> "The Department of Health should reform its regulatory and reimbursement processes to enable therapeutic goods to be registered and reimbursed, by molecular indication in addition to by disease indication. This should include legislative change if necessary."
>
> **Recommendation 13, House of Representatives Standing Committee on Health, Aged Care and Sport, *The New Frontier: Delivering better health for all Australians*[18]**

RECOMMENDATION

Reform Australia's Health Technology Assessment system, including adoption of 'pan-tumour' therapy pathways:

The Government should publish and respond to the recommendations from the HTA Review. As an immediate priority within the reform, it should implement a new pathway in the HTA that:

- assesses precision oncology companion diagnostics and therapies together
- recognises limited clinical data that arise from small patient populations and therefore utilises broader value measures
- accommodates the assessment of multiple indication, genomic focussed applications to expedite access for people with rare and less common cancers.

As an immediate measure, to ensure greater patient access while long-term improvements are put in place, the PBAC should establish decision making frameworks that assess pan-tumour applications for new medicines approval and prioritise applications that will broaden access to medicines already approved for different indications.

3

Pan-tumour, tissue-agnostic therapy approvals are crucial to accelerate access for patients with the rarest cancers and should be a core part of HTA reform. In the interim, the PBAC is already receiving pan-tumour applications and should continue to assess these with urgency.

For example, the PD-1/PD-L1 inhibitor pembrolizumab was first available to Australian patients in September 2015, when it was listed on the PBS for people with metastatic melanoma.

While pembrolizumab is now publicly funded for 12 types of cancer and there are around 3,500 Australians receiving it through the PBS, we estimate that:

At least

3,500
more people

would be eligible for treatment with a PD-1/PD-L1 inhibitor if PBS funding was available for all indications that are currently approved in the United States

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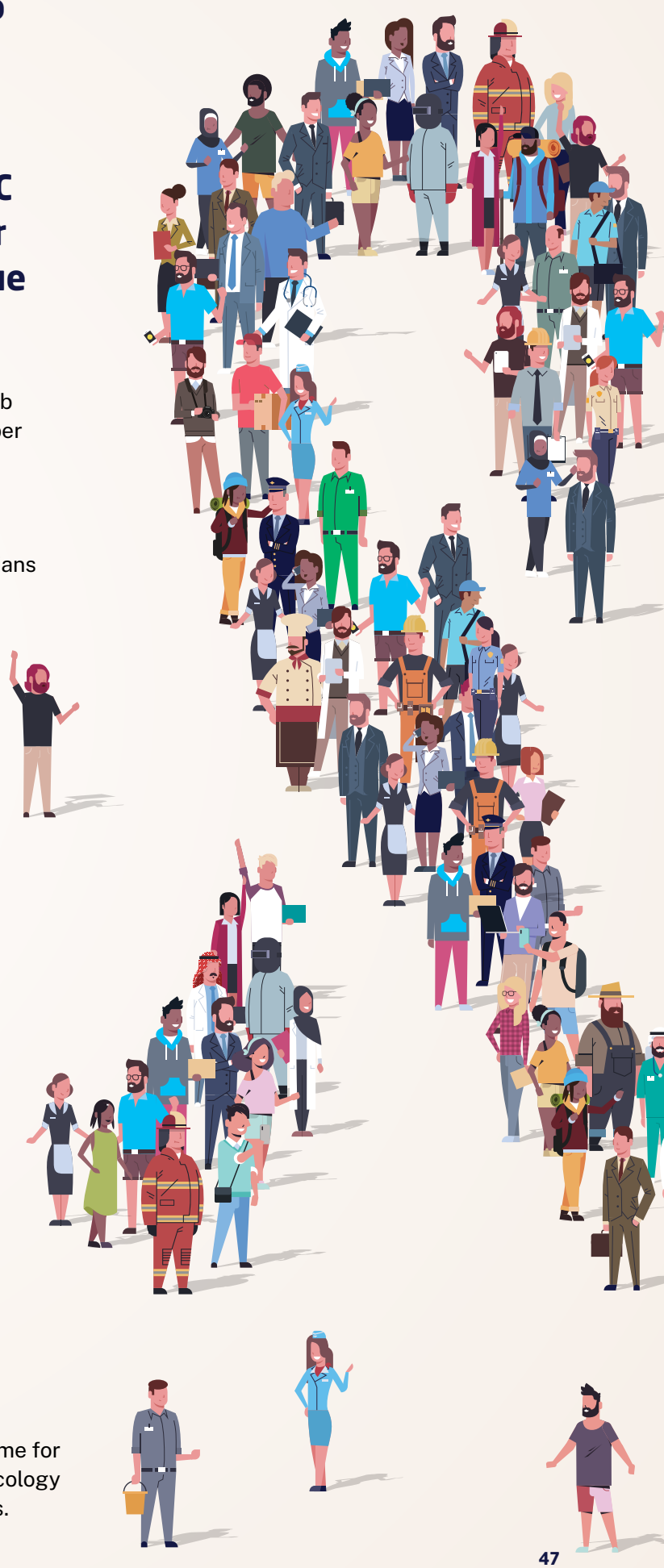
Around

7,000
more people

will be eligible once all remaining indications are eventually approved[21]



Today, there have been seven PD-1/PD-L1 inhibitors approved by the FDA for a range of haematological cancers and solid tumours, namely nivolumab, atezolizumab, avelumab, durvalumab, cemiplimab, dostarlimab and most recently, retifanlimab.[21] It is time for Australia to realise the full potential of precision oncology and deliver equitable access across all cancer types.

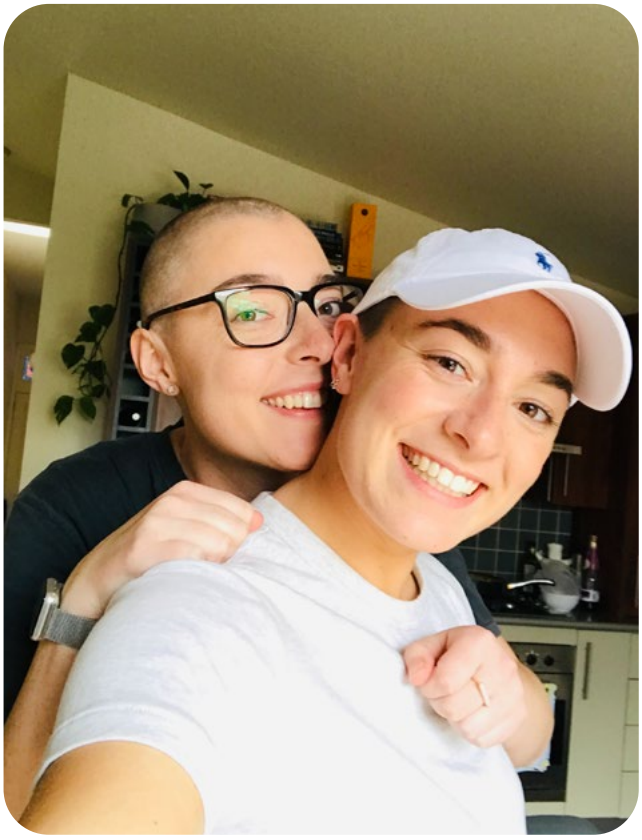
EMILY'S STORY

I am a daughter, a sister, a wife, an aunty and a friend. I am also a person with rare cancer.

In October 2019, I was diagnosed with a rare cancer known as NUT carcinoma, after a tumour the size of a tennis ball was discovered in my sinuses and skull bone. Like many rare cancers, NUT carcinoma is under-recognised by both pathologists and oncologists as it is difficult to diagnose accurately, with approximately 100 people diagnosed each year worldwide. It typically responds poorly to treatment, with an average life expectancy of six-to-nine months.

I underwent chemotherapy, which unfortunately proved ineffective after just two rounds. I then went through a seven-week course of daily radiotherapy with concurrent monthly immunotherapy, which showed some effectiveness over the next 12 months.

At the time of diagnosis, I took part in genomic screening through the Garvin Institute's MoST Research Program. I found the screening process to be overwhelming and lengthy, with little emphasis on the purpose and significance of participating. The testing identified a BRD3-NUT M1 classified genetic mutation, with the recommended treatment being a 'BET inhibitor': a targeted treatment matched to this genetic mutation, with proven effectiveness in some overseas patients. But it was not available in Australia. I was told that access might be possible through the Therapeutic Goods Administration's "Special Access Scheme", should my condition deteriorate further.



As young person diagnosed with a rare cancer and already facing a grim prognosis, I had (and still have) difficulty understanding the inability to access a pre-existing treatment due to Government policy. The treatment, proven effective in similar cases overseas, held the potential to specifically target my tumour. **It represented a potential source of hope and a chance for an improved prognosis – but I could not access it.**

Almost a year after receiving the results of my genomic screening, my cancer spread. I stopped immunotherapy and there were no further treatment options available. Subsequently, my special access application was approved, and I started the BET inhibitor in March 2021. Despite numerous side effects, I remain on the BET Inhibitor today, with additional radiotherapy to manage periodic metastasis.

While my condition is currently considered stable, I still face a future of many unknowns. I will likely never know what it is like to be "cured", "cancer free" or "in remission". Currently, there are no treatment options available if, and when, the BET inhibitor stops working or is discontinued.

There is no denying that cancer is challenging, with a rare cancer diagnosis changing my life in many ways. Despite this, I have been able to draw positives from my experiences. I have a greater appreciation for life and what it means to truly live; I possess an immense gratitude for where I'm at and for the people around me. My connection with loved ones has become stronger and we no longer take quality time together for granted. My sense of purpose has been challenged multiple times and I have come to embrace these changes and opportunities. Facing mortality is tough, however it drives me to live a full and meaningful life, despite living with cancer.

Rare cancers, much like more common cancers, do not discriminate and can affect anyone regardless of circumstance. However, due to their rarity, they present unique challenges for patients and their families.

These include a lack of treatment options, limited availability and strict eligibility criteria for clinical trials, policy and regulatory constraints as well as limited awareness and information. The emotional and psychological impact of a rare cancer diagnosis is substantial, yet there are limited support groups and a lack of patient advocacy and awareness, leading to feelings of isolation.

I want to see a better world for rare, where every person with rare cancer, irrespective of their geographic location, socioeconomic status or background, has fair and equitable access to high-quality health care. This includes specialised and innovative treatment options tailored to their unique needs rather than a “one size fits all” approach. Genomics and precision medicine have come so far, with the potential to unlock otherwise unknown treatment pathways. Raising public awareness about the importance of genomic screening has the potential to empower patients and their families to make informed decisions about their treatment and care. Comprehensive and patient-centred support systems also play a crucial role in supporting the resilience and wellbeing of patients with rare cancers. We should be treated as people, rather than a disease or illness. It is the responsibility of clinicians and health staff to foster environments that promote and encourage shared decision-making, where patients are empowered to actively participate in their care.

Even in a relatively short time, I have seen many positive changes in the rare cancer space. I remain hopeful that with continued effective management in the short to medium-term, I will survive to see curative treatment options for rare cancers like NUT carcinoma made available the future.

Often used as a metaphor for life, just as the sea can be calm or rough with its fair share of ups and downs, so can life. It’s how we ride those waves and how we deal with the challenges that makes a difference.

My sense of purpose has been challenged multiple times and I have come to embrace these changes and opportunities. Facing mortality is tough, however it drives me to live a full and meaningful life, despite living with cancer.

Specialised care and holistic support

Balancing specialised treatment with patient priorities is crucial in cancer care. There is an important balance to strike between centralising highly specialist care for clinical effectiveness and helping people to stay nearer to home and support networks, to give them the best chance of completing and managing their treatment.

Cancer is a chronic disease that requires long-term, person-centred care. People living with cancer, and their families, aim to not only to extend their lives through treatment, but also to enhance their quality while living with cancer. Integrative oncology focuses on helping people achieve the best possible quality of life.

Over 50% of Australians with cancer use complementary therapies to manage their health, cope with side effects, and align their treatment with their cultural and personal values.[58] Integrative care is more prevalent in the U.S., and there are valuable lessons to be learned from their approach.

Evidence shows that integrative and supportive care can improve patient outcomes during cancer treatment and survivorship.[59] To further integrate these practices, we need champions from both clinical and non-clinical backgrounds to lead systemic changes. Additionally, ongoing evaluations of integrative interventions, including qualitative and economic assessments, are crucial for future support and development. These integrative cancer services in Australia are provided in large centres of excellence, but those in rural and regional areas miss out on this emerging field of best practice in holistic, person-centred care.

However, surgery, radiotherapy, and chemotherapy remain the cornerstones of cancer treatment. Innovations are making these treatments more accessible and person-centred. At-home chemotherapy, via tablets or through clinical home visits, has the potential to reduce travel and hospital stays. Hyper-fractionated radiotherapy is becoming more common, reducing the number of treatment sessions and related travel and hospital time, and offering better protection of surrounding healthy tissue.

The rise in the use of telehealth has been a game-changer. Telehealth has the potential to significantly improve access to care for people living with rare and less common cancers residing outside metropolitan areas. While virtual care cannot fully eliminate the need for travel for diagnostic testing or treatment delivery, nor do all patients want to use it, strategic use of telehealth

RARE CANCERS AUSTRALIA

Rare Cancers Australia specialist navigators provide 360-degree personalised support, based on what matters most to the person. This includes:

- emotional support, including connecting with a peer with the same rare diagnosis and monthly support groups
- practical and social support – including transport to treatment and accommodation
- financial support, including help to access superannuation and benefits, crisis payments to cover medical expenses, and food and fuel vouchers
- help to navigate the healthcare system and access information about clinical trials and treatment options
- support to raise funds for genomic sequencing and treatment through our Patient Treatment Fund.

We empower people to navigate and advocate for their own healthcare journey. We use interpreters, link people with culturally sensitive resources and information, and work with partners across the community to provide truly personal support.

can enable patients to access specialised expertise and multidisciplinary care from their local area. Personal support and navigation are particularly suited to delivery via telehealth models, providing person-centred services across settings, beyond the clinical.

Another groundbreaking initiative is the **Australian Rare Cancer (ARC) Portal,** an online platform connecting local clinicians to advice from rare cancer specialists in Australia and around the world.[60] The portal addresses a significant unmet need by enabling patients to be managed by their local clinical team with access to the latest research and clinical advice, including use of genomic testing, recommended treatments, and clinical trials, via a virtual multi-disciplinary team. To-date, 45% of ARC Portal referrals have been for Australians living outside major cities, showing its value for clinicians and patients in rural and remote Australia.[61]

Given Australia's dispersed population, the ARC Portal, as a subsidised service, would have the potential to transform specialised cancer care for people with rare cancers in rural and remote Australia. Importantly, it also collects clinical data to further rare cancer research. However, a critical challenge looms – future funding for the ARC Portal remains uncertain at the time of publication, threatening its capacity to accept referrals.

The Australian Comprehensive Cancer Network (ACCN), one of the first deliverables from the Australian Cancer Plan, has been launched to link patients across Australia, regardless of who they are or where they live, with best available cancer care as close to home as safely possible. The ACCN aims to virtually connect cancer services and multidisciplinary health professionals across public and private health systems with Comprehensive Cancer Centres (CCCs).

These networked models of care, leveraging telehealth and other technologies, are particularly important for improving access for people living in rural and remote areas, including Aboriginal and Torres Strait Islander communities. By enabling connection to specialist centres while remaining on Country, these models help address the geographical and cultural barriers that impact outcomes for Indigenous Australians with rare cancers.

"I live in Northern Tasmania. I have lived here all my life and feel privileged to live in such a beautiful part of our country. I was diagnosed with small cell carcinoma of the ovary hypercalcemic type (SCCOHT), a very rare and aggressive type of ovarian cancer, in 2022. No one here had treated it or knew anything much about it at all and that was terrifying.

The ARC Portal means that I have access to specialists who know about my cancer, who know about the latest research, the international recommendations for treatment. Specialists can have input into my care not just from here in Australia but from all over the world.

I didn't need to travel away from home to see specialists. I could still be treated by my local oncologist who had contact with specialists through the ARC Portal.

The prognosis for someone with my cancer is very poor, however with the right treatment protocols research has shown that the five-year survival rates can be up to 75%. I was able to get the internationally recommended treatment in the recommended timeframe which gives me the best chance of survival.

I have been cancer-free for just over two years now. Two more years of spending time with my family, watching my kids grow, being there when my son got married, meeting my twin granddaughters. Two more years of life I've been given, and I am so grateful."

Amanda, diagnosed with small cell carcinoma of the ovary in 2022

Person-centred support and services

People with rare and less common cancers have higher levels of unmet supportive care needs compared to those with common cancers.[62] The challenges of a rare diagnosis, including isolation, poor prognosis, limited treatment options and a lack of tailored or high-quality information, can exacerbate the difficulties that many people with cancer report in accessing holistic services and support throughout their cancer experience.

There is widespread recognition of the importance and benefits of comprehensive, multi-disciplinary care that is personalised to the individual and their preferences.[63] This is important at every stage of the cancer experience – from first symptoms of cancer through to survivorship and end-of-life care. Care and support models must be designed around the whole person, and not just their cancer. They must also be tailored and accessible for different community groups. For Aboriginal and Torres Strait Islander peoples, culturally safe cancer care is essential, incorporating Indigenous knowledge and perspectives on health and wellbeing. This includes partnering with Aboriginal Community Controlled Health Services, increasing the Indigenous cancer workforce, and building cultural competency across cancer services.

A key component of holistic care is shared decision- making, which enables people with cancer and their carers to make informed decisions about their clinical care, based on comprehensive information about medical facts and treatment options balanced with what matters to the individual.[64, 65] Done well, this approach ensures people are fully informed of their treatment options and the trade-offs between risks and benefits, and that their values and preferences are incorporated into the decision.[66, 67] More informed cancer patients are more engaged and more likely to fully deliberate about the risks and benefit trade-offs of the different treatment options, and they are more satisfied with the clinical experience.[66] There is also evidence that more informed and involved cancer patients have better psychosocial, and in some cases physical, outcomes.[68]

RECOMMENDATION

Invest in novel clinical trials and telehealth:

Government, healthcare providers and industry partners should invest in novel clinical trial designs and telehealth infrastructure to expand access to precision oncology for rare cancers and patients in rural and regional areas.

4

The **Australian Cancer Nursing and Navigation Program (ACNNP)** is a key component of the Australian Cancer Plan's 10-year ambition that "people affected by cancer are partners in culturally safe, equitable and responsive cancer care, and health services and systems are trusted and supported for optimal experience, quality of life and cancer outcomes".

$166 million has been granted to ensure all people with cancer have access to high quality and culturally safe care, irrespective of their cancer type or location.

Rare Cancers Australia is delighted to be a partner in this initiative, which allows a rapid expansion of our personalised patient support and navigation, to keep pace with rising demand.

Crucially, through the ACNNP, Government and patient organisations are working together towards the same goals, sharing information and resources in a coordinated approach, with measurable outcomes. As well as direct patient support, this program should provide a deeper insight into patient needs and service gaps along the whole cancer pathway, contributing to long-term and sustainable support and service solutions.

Complete and representative data

Comprehensive, high-quality data are the foundation for advancing rare cancer research, prevention, diagnosis, treatment, and care delivery. Accurate data collection and timely reporting are vital for understanding the prevalence, incidence, and outcomes of all cancers, and what is happening at a patient, system, and national level. This enables recognition of patterns and disparities, providing crucial insights into intervention opportunities that might otherwise remain obscured.

Epidemiological data

Historically, rare cancer data availability and quality have been poor. This is in part due to the challenges of accurately coding and reporting rare cancers, with small patient numbers that are geographically dispersed and have diagnostic complexity. Variation in jurisdictional cancer registries also limits national reporting. Key indicators, such as stage of diagnosis, are currently routinely captured only for the five most commonly diagnosed cancers: melanoma, breast, prostate, lung, and colorectal cancers. This results in a significant gap in our knowledge of rare and less common cancers.

Establishing a comprehensive cancer data framework for Australia, with a defined minimum data set to include rare and less common cancers, is imperative.

The European Rare Cancer Network (RARECAREnet) and the US National Cancer Institute's Rare Tumor Patient Registry offer models for multi-institutional data collaboration and patient engagement in rare cancer research. These initiatives have demonstrated the feasibility and impact of coordinated rare cancer data efforts and offer valuable learnings for the Australian context.

"We need a cancer data framework for Australia, and we need a minimum data set for collection. The data needs to be end-to-end, not just the bit where you're in the mainstream health system."

Professor Dorothy Keefe, Chief Executive Officer of Cancer Australia; Committee Hansard, 1 March 2024[6]

Patient organisations are united in a quest for data to drive outcomes. Following strong, patient-led campaigning from Breast Cancer Network Australia, the Government recognised the value of better cancer data on stage and recurrence with $1.5 million funding to the AIHW for an Australian Cancer Data Alliance.[69] This will support state and territory cancer registries to work towards routinely collecting cancer stage and recurrence data. This is an important step that we must build from.

> "This funding is a significant step forward and will help the sector lead and pioneer the collection of these important data to inform and drive policy, innovation, planning, treatment and care. Now, we can begin consolidating a way forward for better quality data, not just for breast cancer, but for all metastatic cancers."
>
> **Vicki Durston, Director of Policy, Advocacy and Support Services, Breast Cancer Network Australia[69]**

Genomic data

To enable widespread use of precision medicine, supported by comprehensive genomic profiling, we must prioritise genomic data collection. This information can predict how a person's cancer will respond to surgery or drug therapy and guides the use of existing treatments or suggests more targeted treatments. We need technologies and systems capable of efficiently collating and interpreting data, and providing fast, easily accessible outputs to help clinicians discuss results and matched therapy options with their patients.

Patient-reported outcome and experience measures

To deliver truly person-centred support and care, it is essential that the voices and lived experiences of people with rare cancers and their families are at the heart of service design and delivery. One powerful way to achieve this is through the routine collection and use of patient-reported outcome measures (PROMs) and patient-reported experience measures (PREMs) in rare cancer research and care.

PROMs are measures that capture a patient's own assessment of their symptoms, functional status, and quality of life. PREMs encompass a broader range of patient-reported data, including narratives, satisfaction scores, and feedback on specific aspects of care. Together, they provide valuable insights into the real-world impact of cancer and its treatment on people's lives, and can help to identify unmet needs, guide supportive care interventions, and inform patient-centred service improvements.[53] PROMs and PREMs are especially important for understanding the unique perspectives and needs of people from priority populations. Language barriers, lack of trust in mainstream health services, cultural beliefs about cancer and health literacy can significantly impact engagement with cancer services and the support a person may need.

Some states and services are using PROMs and PREMs to measure and understand patient needs and shape their services. To realise the Australian Cancer Plan's ambition of equity, we need a national view of experience across the cancer pathway, from screening and diagnosis through to survivorship and end of life care. It should be broken down by cancer type, including rare and less common types, and by priority population. This is a critical part of achieving "integrated, coordinated, data-driven, high-quality health service systems that consistently deliver optimal cancer care and excellence in outcomes".[1]

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Rare Cancers Australia is commissioning the first *national rare cancer lived experience survey* to better understand the needs of people living with rare and less common cancers, and their carers. The data will identify unmet needs to help Rare Cancers Australia and health commissioners to design and target support services.

Data-driven service delivery

Accurate and timely data are also critical for driving better clinical outcomes and service improvement, identifying variation and best practice across the country and internationally. Initiatives such as the International Cancer Benchmarking Partnership[70] (NSW, Victoria and Western Australia participate) help to understand differences in population health and system design that can unlock innovative approaches. Benchmarking within systems and nationally, as seen in *Treating people with cancer in Queensland and Victoria: National Benchmarking Starts Here,*[71] should become the norm and all states and territories should be using clinical and patient-reported data to assess and improve their services.

Artificial intelligence (AI) has the potential to enhance epidemiological, genomic, and service delivery data capture and analysis. AI algorithms can rapidly process vast amounts of data from multiple sources, identify patterns and predict outcomes in ways that traditional methods cannot. However, significant investment in data infrastructure, governance frameworks and workforce capabilities are needed to realise the full potential of AI in cancer.

RECOMMENDATION

Ensure rare and less common cancers are represented within the National Cancer Data Framework.

Cancer Australia and the AIHW should:

- prioritise the rapid development and implementation of an ambitious National Cancer Data Framework and minimum cancer data set, building on the strengths of state registries and enabling a more timely, accurate and person-centred national view of cancer pathways and outcomes for rare and less common cancers
- work with states and territories to ensure all cancer registries are capturing timely, high-quality and consistent data across the cancer pathway, including molecular sub-type and stage at diagnosis and indigenous status, for all cancer subtypes.

5

Rapid translation of research into clinical practice

For a long time, rare cancer research was underfunded and not prioritised. Targeted investment from the NHMRC and the MRFF, particularly over the past five years, has shifted the focus and driven a much-needed increase in research opportunities.

Initiatives such as the MRFF Low Survival Cancers Mission and Reducing Health Inequities Mission will be transformational for people with rare and less common cancers,[72] and we are already seeing direct patient benefit from genomic screening and precision oncology studies.

However, we have a long way to go. Increased and sustained investment in rare cancer, genomic and precision oncology research is the key to tackling the poor outcomes, with a collaborative approach to bridging the gap between research and clinical practice, is the key to finally improving rare and less common cancer outcomes.

Translational research

There are significant challenges in translating advances in research and development into tangible, timely benefits for patients. The reasons for this lag are complex, including inflexible regulatory and reimbursement pathways, lack of commercial incentives for rare cancer drug development, and limited health system capacity to implement new models of precision oncology equitably.[73]

There is an urgent need to accelerate the pace of change and ensure that hard-won research advances deliver meaningful benefits for people with rare and less common cancers. This requires strategic, coordinated efforts to:

- boost dedicated rare cancer research funding across the pipeline and foster collaborative research networks and data-sharing platforms to maximise collective impact
- streamline regulatory and HTA processes to enable earlier access to emerging therapies.
- invest in health system capacity to equitably implement new care models and facilitate clinical trial access
- enable and support healthcare systems to update clinical practice and patient pathways quickly and safely.

The goal is to create a seamless and efficient pipeline that rapidly moves breakthrough discoveries from bench to bedside.

A Rare Cancer Moonshot requires a research workforce and infrastructure that can drive the rapid translation of discoveries into practice. Investment in state-of-the-art research facilities, biobanks, genomic sequencing capacity and data systems are critical to support innovative rare cancer research in Australia. Equally important is ensuring the right training programs and career pathways that attract and retain top talent in rare cancer research, from basic scientists to clinical trialists, and building capacity and expertise across health systems for research implementation.

RECOMMENDATION

Prioritise research funding with a focus on translation into clinical practice:

The NHMRC and the MRFF should prioritise research into rare cancers, genomics, the application of artificial intelligence, innovative testing and treatment, and critically, its translation into clinical practice.

6

The NHMRC committed

$221.6 million

for 186 grants relevant to rare and less common cancers between 2018 and 2022

The MRFF has invested

$37.9 million

across 25 rare cancer-specific research grants since its inception in 2015.[6]

In the 2024 Federal Budget, additional research was funded via the MRFF for a

new 10-Year Low Survival Cancers Mission and a new 10-Year Reducing Health Inequities Mission.[10]

An audit conducted by Cancer Australia on cancer research funding revealed that between 2012 and 2020, over

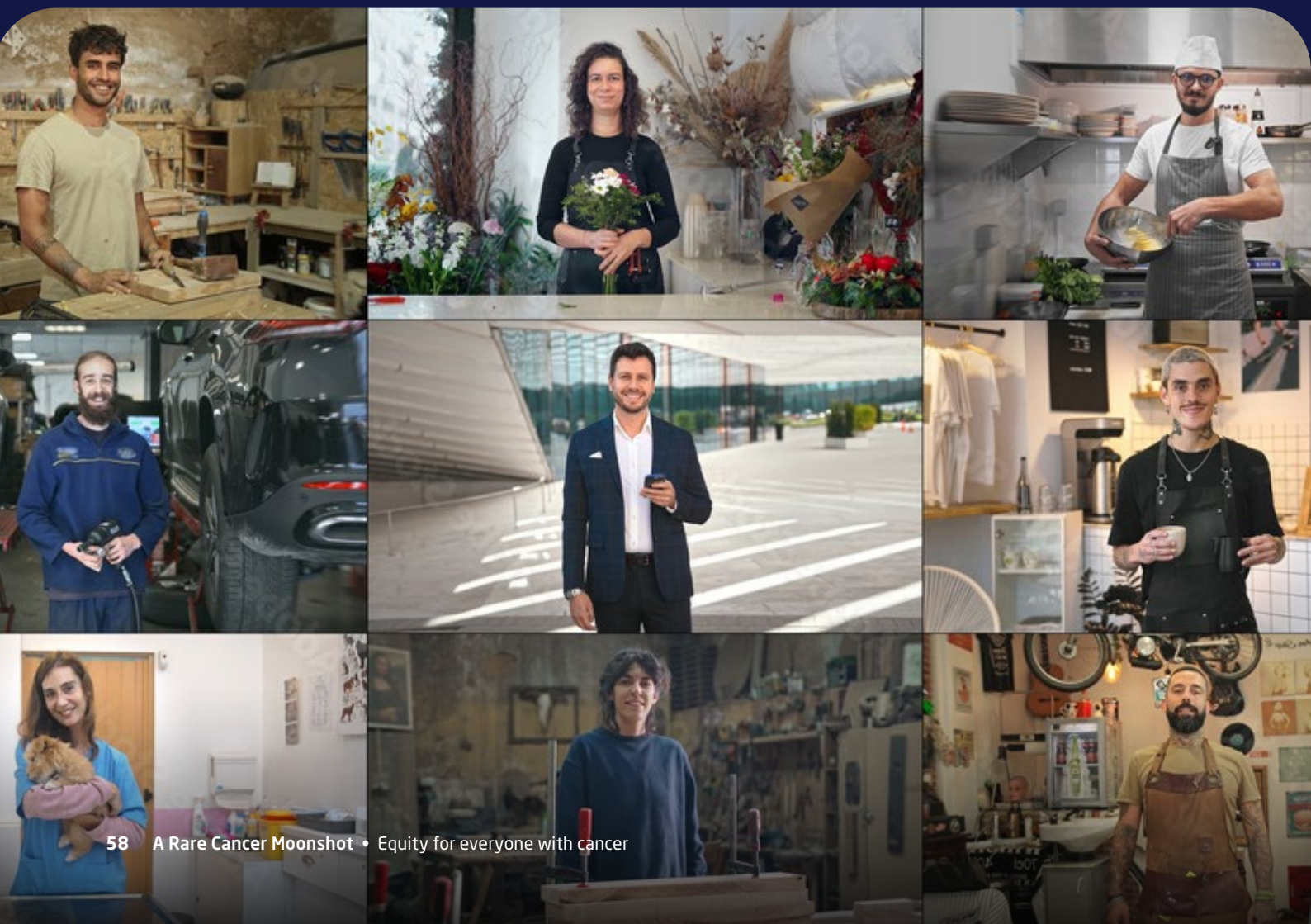
4,800

cancer research projects and programs

were funded across Australia, with a total value of $2.12 billion.[74]

This funding has supported crucial projects spanning the research continuum, from understanding the molecular drivers and novel therapeutic targets in rare cancers, to advancing precision medicine approaches and testing innovative models of care.

The countdown is on



We have an unprecedented opportunity to transform rare cancer outcomes and experiences. The scientific, policy and community momentum is there. Now is the time for coordinated, ambitious action.

By uniting behind a Rare Cancer Moonshot we can create a future where, by 2035:

- more than 90% of people survive at least five years after diagnosis, regardless of rarity
- there is true equity of experience for everyone with cancer.

We can realise a future where everyone with cancer benefits equally from advances in science and has equitable access to world-class care. Where diagnosis is swift, treatment is targeted, and no one faces this challenge alone.

The immediate priorities to launch the Moonshot are:

1. **Endorse the recommendations of the Senate inquiry into Equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer.**
2. **Develop and implement the National Genomics Strategy.**
3. **Reform Australia's Health Technology Assessment system.**
4. **Invest in novel clinical trials and telehealth.**
5. **Accelerate the National Cancer Data Framework.**
6. **Prioritise research funding with a focus on translation into clinical practice.**

Join us as we strive for equity of outcomes for people with rare and less common cancers. Together, we can defy the odds and bring new hope to the patients of today and tomorrow.

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Join us as we strive for equity of outcomes for people with rare and less common cancers. Together, we can defy the odds and bring new hope to the patients of today and tomorrow.


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