

Building a Future of Life-Saving Clinical Trials for Children with Cancer

National Paediatric Oncology
Industry-sponsored Clinical Trial Roundtable





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Foreword

When a child is diagnosed with cancer, families are confronted with life-changing uncertainty. At such a vulnerable time, every child deserves access to the best possible care. For some, clinical trials can offer hope where few alternative treatment options are available. Historically, enrolment on clinical trials, notably international phase 3 trials, has informed standard of care and been the cornerstone for improving survival and outcomes for a number of childhood cancer types. Whilst incredible improvements have been made for some cancers, far too many children succumb to their disease or are left with long-term side effects of treatment. Clinical trials at all phases of the drug development pathway not only inform care, but can offer hope to those families where curative treatment options are limited.

Children's cancer services in Australia are among the best in the world. In each children's hospital in Australia, a network of highly dedicated, multidisciplinary healthcare professionals is centred around optimising the care provided to each patient. We are among the most successful nations in establishing clinical trials for children with cancer, which is driven by Cancer Australia's funded cooperative clinical trials group for childhood cancer: the Australian and New Zealand Children's Haematology/Oncology Group (ANZCHOG). This provides a solid foundation for the delivery of high-quality clinical trials in Australia.

The Zero Childhood Cancer Program (ZERO) is a world-leading precision medicine program that provides comprehensive genomic profiling for Australian children with cancer, with the program expanding to include adolescents and young adults (AYA) aged 19–25 with paediatric-type cancers.

Industry-sponsored clinical trials play an important role in enabling access to innovative therapies. Investments from sponsors contribute to the Australian clinical trials ecosystem and build our scientific know-how as a foundation for future research and development.

The National Paediatric Oncology Industry-sponsored Clinical Trial Roundtable brought together leaders from patient advocacy organisations, clinical care, research, industry and government to identify the changes needed to position Australia as a leading destination for industry-sponsored paediatric oncology clinical trials and improve access to trials for children with cancer.

The breadth of stakeholders represented reflects the reality that improving access to innovative therapies cannot be achieved by any one organisation alone but instead is a collective effort across the whole ecosystem. Through these discussions, participants found alignment on key actions needed to create a more attractive destination for industry-sponsored clinical trials as well as opportunities to overcome access barriers for patients and families.

This white paper reflects the collective insights from that discussion and the shared ambition to create a more connected ecosystem that is internationally competitive and strives to deliver the best outcomes for Australian children and their families.



A handwritten signature in black ink, appearing to read 'L Chesler'.

Professor Louis Chesler
Executive Director
Children's Cancer Institute



A handwritten signature in black ink, appearing to read 'Liz de Somer'.

Liz de Somer
Chief Executive Officer
Medicines Australia



A handwritten signature in black ink, appearing to read 'Andy Moore'.

Assoc. Prof. Andy Moore
Chair
Australian and New Zealand
Children's Haematology/
Oncology Group

Acknowledgements

Biointelect, the Children's Cancer Institute and Medicines Australia wish to acknowledge the ongoing support and participation of the following organisations and individuals who have contributed to this work, from the roundtable to the development of this paper.

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* Unable to attend the roundtable but subsequently contributed insights through a separate consultation process.

This white paper reflects the views expressed by participants at the National Paediatric Oncology Clinical Trial Roundtable and does not necessarily represent the views of Children's Cancer Institute, Medicines Australia or the organisations associated with roundtable participants.

Executive Summary



In Australia, around 1,000 children are diagnosed with cancer each year, representing around 1% of all cancer diagnoses¹.

At any given time, more than 2,000 children are undergoing cancer treatment and, tragically, each year 200 of these children will not survive.

Although cancer is rare in children, it is the leading cause of disease-related death in children.



Participating in clinical trials is a critical path to accessing the latest global therapeutic innovations for Australian children with cancer. International cooperative group clinical trials enable access to novel therapies and treatment protocols for many children. Attracting more industry-sponsored trials would mean giving Australian children more opportunities to access potentially life-saving treatments.

Converting existing clinical trial capability into greater access to paediatric oncology innovation

Paediatric cancers are rare diseases and therapies for paediatric cancers are frequently designated as orphan drugs. Regulators and reimbursement agencies may waive fees or offer advantages such as exclusivity, where no other medicine will be approved over a specific period. This aims to address the commercial challenges of investing in the development of new therapies for paediatric cancers. In the broader oncology context, the commercial incentive to invest in the development of adult therapies is significantly greater than for paediatric, which is reflected in the number of trials conducted globally and in Australia.

Australia has a world-class paediatric clinical trials ecosystem that supports a mix of investigator-led, cooperative group and industry-sponsored trials. Our performance in attracting adult oncology trials, with more than twice the number of trial start-ups per capita than the US, provides a clear benchmark for paediatric trials. Through ANZCHOG, a high-quality, mobilised national network already exists for paediatric trials, which could be leveraged to increase the number of industry-sponsored paediatric trials in Australia.



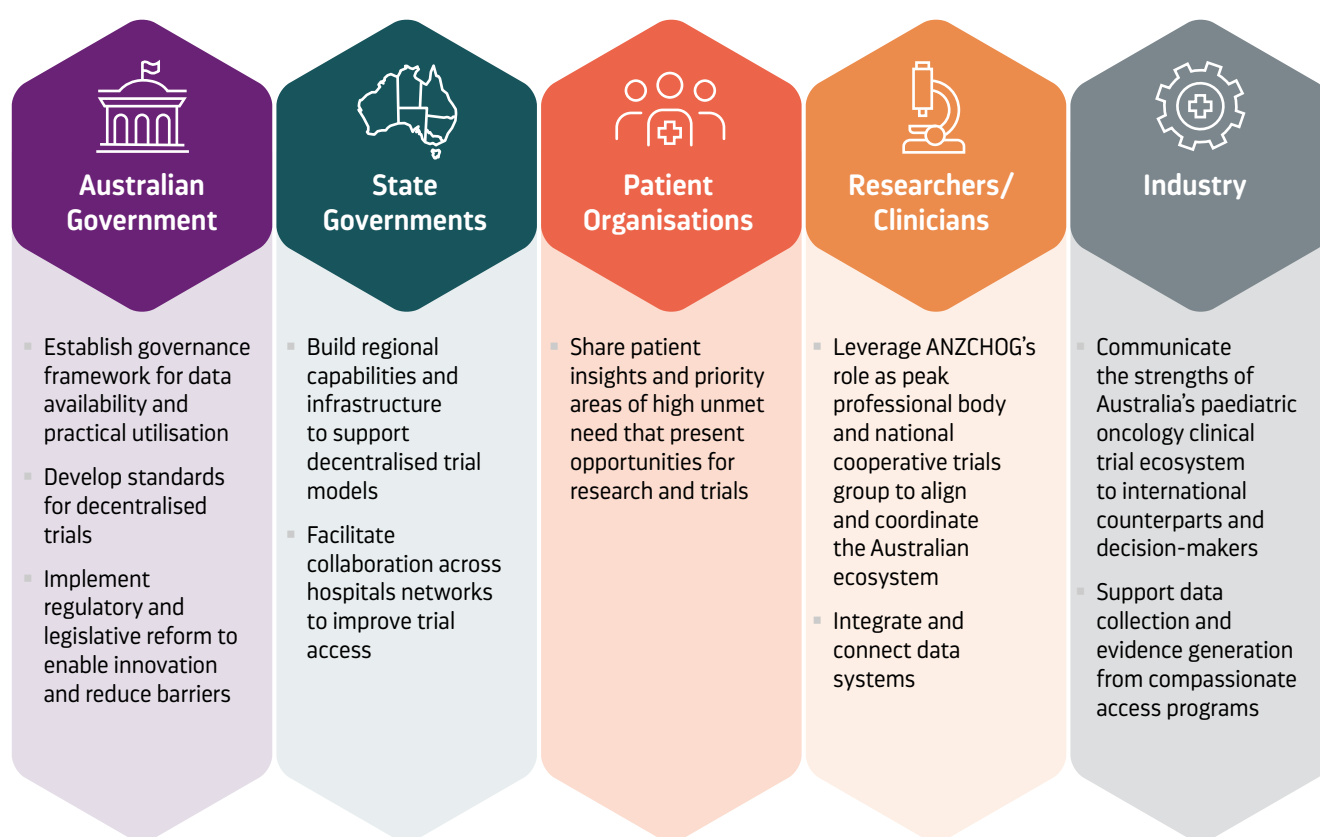
National Paediatric Oncology Clinical Trial Roundtable

The Children's Cancer Institute (CCI) and Medicines Australia's Oncology Industry Taskforce convened a roundtable at Minderoo Children's Comprehensive Cancer Centre in July 2026 to identify a way forward for Australia to attract industry-sponsored clinical trials for children with cancer. The roundtable was attended by 18 leaders from patient advocacy groups, clinicians, researchers, government and industry. Discussions focused on:

- The current role of industry-sponsored paediatric oncology trials in Australia.
- Key areas requiring support and intervention to overcome existing barriers to industry-sponsored trial activity and patient access.
- Priority actions required to attract greater industry investment and improve patient access to clinical trial opportunities.

Four core recommendations arose from the discussions at the roundtable.

Figure 1: Key stakeholder groups and priority actions required to strengthen Australia's paediatric oncology clinical trial ecosystem



A roadmap to expand clinical trial opportunities for children with cancer

Access to clinical trials is crucial for Australian children with cancer who have limited treatment options. In an increasingly competitive international clinical trial landscape, there is a need for ongoing review and commitment to continuous improvement to remain globally competitive. Building on existing strengths and addressing barriers with a mobilised stakeholder community, we can continue to offer expanded trial opportunities for Australian children with cancer.

1



Create a nationally coordinated consortium to align investment, advocacy and sponsor engagement activities across Australia's paediatric oncology ecosystem

The paediatric oncology ecosystem in Australia is supported by an established network of researchers, clinicians, patient organisations and programs like the Zero Childhood Cancer Program (ZERO) and ANZCHOG that enable world class clinical trials. However, the ecosystem is under-resourced and fragmented across institutions and jurisdictions, leading to inefficiencies and a lack of visibility for international sponsors to understand Australia's true clinical trial capabilities.

ANZCHOG is well placed to lead a nationally coordinated consortium to align stakeholders around shared priorities. This would reduce duplication of effort, optimise the value of investments and infrastructure and generate a clear value proposition for global decision-makers to engage and support more consistent trial activation.

2



Build on existing data infrastructure to enhance the utilisation of data and create a more connected and collaborative ecosystem that improves patient care

Australia has a range of data assets that add significant value to the clinical trials ecosystem. In paediatric oncology, the ZERO program and platforms such as CIRCAA and SACHA enable data collection at key points in patient journeys.

Rather than building entirely new systems, there is an opportunity to better utilise existing data by integrating these sources from diagnosis through treatment and patient outcomes. Greater connectivity would provide clinicians and researchers access to data to inform decision-making. It could also support clinical trial recruitment and, potentially, enable generation of evidence on the safety, efficacy and real-world use of therapies for sponsors.

Establishing nationally consistent governance for access and the use of data is key to unlocking these benefits.

3



Expand equitable access through decentralised trial delivery and strengthened site capabilities

Australia's geography is a major barrier, both for patients in accessing trials and for sponsors recruiting for trials in relatively small populations. Expanding decentralised trial models, including teletrials and shared-care approaches, would allow more children to access innovative therapies closer to home, while reducing the burden of participating in trials on families and improving recruitment outcomes.

There are opportunities to reduce travel burden, while maintaining specialist oversight, by combining satellite trials with a standardised trial activation process that leverages regional services in support of specialist paediatric cancer centres. While implementation of these approaches remains at an early stage, experience from decentralised trials in adult oncology demonstrates the potential for regional sites to support metropolitan cancer centres.

Any application within paediatric oncology should recognise the distinct clinical requirements of children and young people and be tailored accordingly.

4



Align commercial incentives with the realities of paediatric oncology development, including regulatory and reimbursement frameworks to provide a pathway to market following clinical trials

A company's decision to conduct a clinical trial in Australia is aligned with commercial realities, including expectations of the path to market following clinical trials. Paediatric medicines are typically designated as orphan drugs by international regulators, which confers special considerations through regulatory and reimbursement processes.

International case studies for enabling launches of paediatric medicines include market protection and fast-tracked pathways. Where possible, giving appropriate consideration to patient safety, regulatory and reimbursement frameworks should accommodate the unique challenges for paediatric oncology.

Australia's Pharmaceutical Benefits Advisory Committee (PBAC) has been shown to offer greater flexibility in approving paediatric medicines, and this could be formalised. Adopting Recommendation 2 of the Health Technology Assessment Policy and Methods Review would prioritise age-agnostic PBS listings.

Introduction

In Australia, around 1,000 children are diagnosed with cancer each year, representing around 1% of all cancer diagnoses². Although cancer is rare in children, it is the second leading cause of death among children³ and the leading cause of disease-related death.

Survival rates in childhood cancer have improved significantly.



The five-year survival rate for children aged 0–14 diagnosed with cancer in Australia was approximately 86% over 2007–2016, improving from 73% in the previous decade⁴.

The development of new treatments has been a major driver of this improvement.



Two-thirds of childhood cancer survivors experience severe life-long late effects of treatment.

The significant toxicity of cancer treatments has profound impacts on developing bodies. These place substantial burden on the lives of survivors, their families and the healthcare system.

Access to more targeted and less toxic therapies is vital to address the long-term burden of survivorship⁵.

For Australian children with limited treatment options, participating in clinical trials is a critical path to accessing the latest global therapeutic innovations.

Childhood cancers are rare and comprise multiple small, biologically distinct patient populations. This creates challenges for patient recruitment to clinical trials, which are compounded by limited commercial incentives to invest in the development of paediatric cancer therapies. Globally, there are few paediatric oncology clinical trials, relative to adult oncology trials, and competition to attract global trials to Australian sites is strong. Overcoming these barriers is critical to expanding access to clinical trials for Australian children with cancer.



The National Paediatric Oncology Industry-sponsored Clinical Trial Roundtable

The Children's Cancer Institute and Medicines Australia's Oncology Industry Taskforce convened a roundtable at Minderoo Children's Comprehensive Cancer Centre in July 2026 to identify a way forward for Australia to remain a world leader in attracting industry-sponsored clinical trials for children with cancer.

Bringing together stakeholders from patient advocacy groups, clinicians, researchers, government and industry, the roundtable sought to identify opportunities and develop practical recommendations to strengthen Australia's paediatric oncology clinical trial ecosystem and improve access to innovative therapies, through clinical trials, for patients.

Discussions focused on putting patients first and examined Australia's paediatric clinical trials ecosystem, across infrastructure, organisations and key roles. Participants explored the factors across the ecosystem from motivations to open clinical trial sites to systemic barriers that limit patient participation, considering the intersection of the policy landscape with commercial realities. At the heart of this discussion was a commitment to improve access to the best medicines available globally and clinical trial opportunities for Australian children with cancer.

The objective of the roundtable was to develop a compelling, evidence-based case for changes needed to position Australia as a leading destination for industry-sponsored paediatric oncology clinical trials. Through collaborative discussions, participants sought to identify practical recommendations addressed to government, regulators, industry and the clinical community.

Australia is among the world's leading countries for paediatric oncology trials

Australia has a strong paediatric oncology clinical trials ecosystem, despite our relatively small and geographically dispersed population. Australia is among the leading countries in the world for paediatric oncology trials, with sites for 9.4% of all global trials⁶ between 1987 and 2024. The majority of these are investigator-led trials, and 25%–33% of paediatric oncology trials are industry sponsored^{7,8}.

The success of investigator-led, cooperative group clinical trials in Australia highlights the strength of the existing ecosystem, the support of funders and our ability to deliver world-class clinical trials. The capability to deliver trials already exists. The opportunity now is to understand the practical and commercial barriers that influence sponsor decisions to include Australian sites in global trials, and to unlock greater participation in global paediatric oncology development.

Figure 2: Primary sponsor frequency by country for paediatric only clinical trials (n=3149).



Reproduced from Mikkelsen et. al⁹

Current State Assessment

Australia’s world-leading performance in adult oncology trials is not reflected in paediatric oncology

Australia is among the world leaders in attracting industry-sponsored oncology trials. Figure 3 shows that, on a per capita basis, Australian sites were more than twice as likely to be selected for global adult trials than even the United States (US) over the last decade¹⁰. We have consistently outperformed many comparable nations on a per capita basis and routinely capture 5% of global industry-sponsored trials.

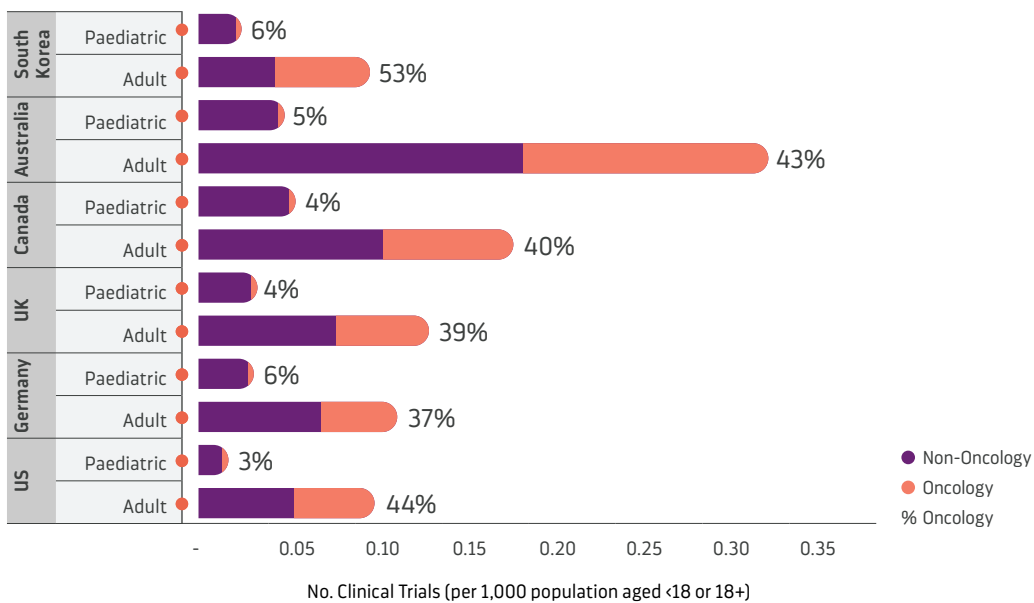
As a leading destination for clinical trials globally, Australia benefits from substantial direct foreign investment, which supports highly skilled employment in the clinical trials sector and drives economic growth. In 2022, \$1.6 billion¹¹ was spent on clinical trials activity in Australia with earlier analysis from 2018 describing over 60% of annual clinical trial expenditure attributed to foreign investment¹². This highlights the significant economic contribution of internationally sponsored clinical research, supporting productivity, workforce capability and continued investment in Australia’s clinical trials ecosystem.

This extraordinary success in adult trials has not been replicated in paediatric oncology, where Australia’s performance is broadly comparable to other developed nations. In absolute numbers, this means that there are few trial opportunities for children with cancer.

In Australia, 75% of paediatric oncology trials are not industry-sponsored. In 2024 (latest available data), ANZCHOG* had 31 trials open or in follow-up with 892 enrolments alongside other investigator-led initiatives¹³ whereas, in the 12 months to June 2026, there were only seven active industry-sponsored clinical trials¹⁴.

*See further details about ANZCHOG in the section: Australian researchers and networks enable access to clinical trials.

Figure 3: Industry-sponsored oncology clinical trials per capita by country, comparing adult and paediatric populations (2015–2026)



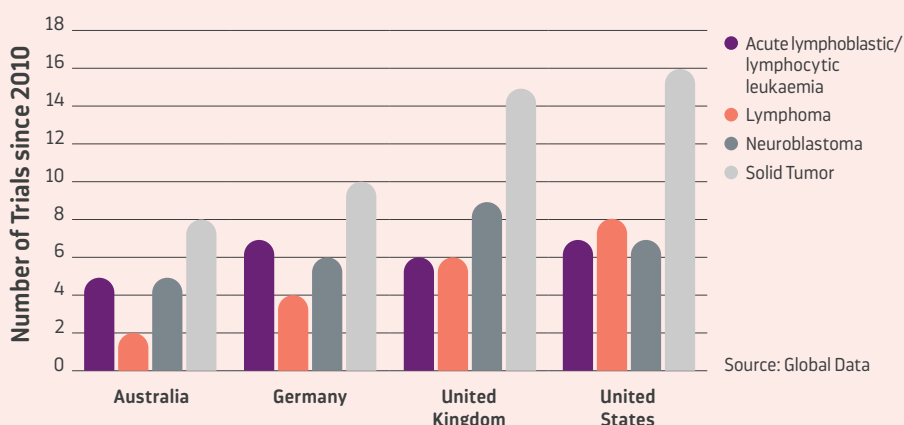
Source: Global Data ¹⁵

Note: Search criteria included trials with start date 1 January 2015 to 11 June 2026; sponsor type: company; study type: interventional.

Trial Availability by Indication

A comparison of clinical trials by disease type demonstrates substantial variation across different indications when comparing Australia to the US, UK and Germany. While some indications have multiple active studies, patients diagnosed with diseases such as neuroblastoma, lymphoma and ALL have access to fewer trial opportunities in Australia than in Europe and the US.

Key Paediatric Clinical Trial Indications



The resulting disparities in access to precision medicine modalities, including CAR-T, new diagnostics and treatments, place a substantial burden on families to travel overseas and pay for potentially life saving treatments.

Global Competitiveness is Critical to Expanding Access to Innovative Therapies

Global sponsors of paediatric trials can bring the latest innovative therapies to Australian children with cancer. While investigator-led studies provide important opportunities for children to access novel therapies, they are often limited in their translation to broader commercial pathways via regulatory approvals or funded access pathways and reimbursement.

Clinical trials that are conducted in Australia are typically part of a larger global study. Industry participants confirmed that the decision regarding site selection is generally made by sponsors at a global level. Site selection is heavily influenced by the feasibility of delivering the study, in particular times for startup, recruitment and completion, as well as the quality of the study. Consideration is also given to whether patients will have a clear pathway to ongoing access following trial completion, reflecting both ethical obligations to participants and the commercial incentive to invest in Australia.

Table 1: Key factors influencing global sponsor decisions on clinical trial site selection from roundtable discussion and consultations.

Patient Access & Recruitment	Trial Delivery Capability	Commercial and Financial Attractiveness
Patient identification: Precision medicine programs, registries and screening platforms support rapid identification of eligible patients.	Trial start-up speed: Fast activation provides sites/countries with competitive advantage	Cost of trial delivery: Overall cost-effectiveness of conducting trials, relative to other countries.
Recruitment speed: Companies need to be confident that patients can be enrolled efficiently.	Investigator experience: Experienced investigators provide confidence in protocol adherence and participant management.	Market access potential: Supportive pathway from clinical development to patient access.
Patient access: Ability for patients to access trial sites, including overcoming geographic and logistical barriers.	Regulatory and governance efficiency: Streamlined ethics, governance and regulatory processes that reduce administrative burden and accelerate trial activation.	Industry partnerships: Strong industry-clinician-research collaborations increase confidence in the local ecosystem.
	Site capacity and infrastructure: Appropriate staff, facilities and operational support to deliver trial outcomes.	

National Policy Snapshot

Government policy plays an important role in aligning Australia's objectives for clinical trials and shaping our competitiveness as a destination for industry-sponsored trials. As paediatric oncology trials are typically part of multinational development programs, Australia's ability to compete for trials depends not only on scientific excellence, but also on providing a predictable, efficient and commercially attractive environment for research investment.

The Ambitious Australia Report is an independent review of the challenges facing Australia's research and development sector. The report highlights the need to strengthen commercial incentives for R&D as well as increase investment and capital to fuel innovation. It calls for government leadership to simplify and coordinate the research ecosystem.

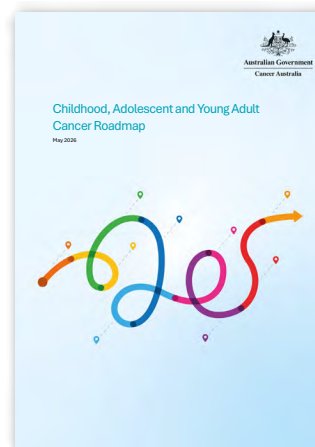
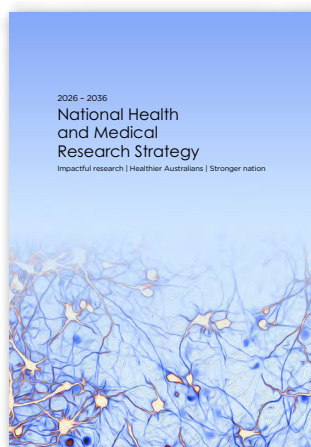
The National Health and Medical Research Strategy provides a 10-year vision for strengthening Australia's research ecosystem. While not specific to paediatric oncology, this initiative establishes a framework for researchers, clinicians, policymakers and industry leaders to collaborate on reforms that could also support the continued growth of the paediatric oncology ecosystem.

The Childhood, Adolescent and Young Adult Cancer Roadmap recognises the need for a more coordinated and sustainable approach to childhood cancer research and clinical trials. It identifies the need for collaboration between research and industry to improve future innovation and patient outcomes through access to clinical trials, strengthening the workforce and developing an integrated data ecosystem as key priorities.

The **National One Stop Shop (NOSS)** is a national reform aiming to streamline clinical trials approvals and make it easier for clinicians and patients to find and conduct clinical trials in Australia. NOSS offers a coordinated approach to ethics, governance and site activation processes to improve efficiency for sponsors and trial sites. While the significant potential of NOSS is clear, roundtable participants felt that further implementation and alignment across jurisdictions will be required to fully realise its benefits in the context of paediatric oncology trials.

The **Clinical Trial Notification (CTN)** scheme is a key component of Australia's clinical trial framework. Under the CTN pathway, sponsors can expedite their trial start up by commencing Human Research Ethics Committee (HREC) review, rather than seeking formal approval from the TGA (however the TGA is still notified of the trial through the CTN). This avenue is a competitive advantage because it reduces administrative burden, especially for early phase trials. There are similar schemes internationally, so while CTN is important, it may not confer a distinct advantage for Australia.

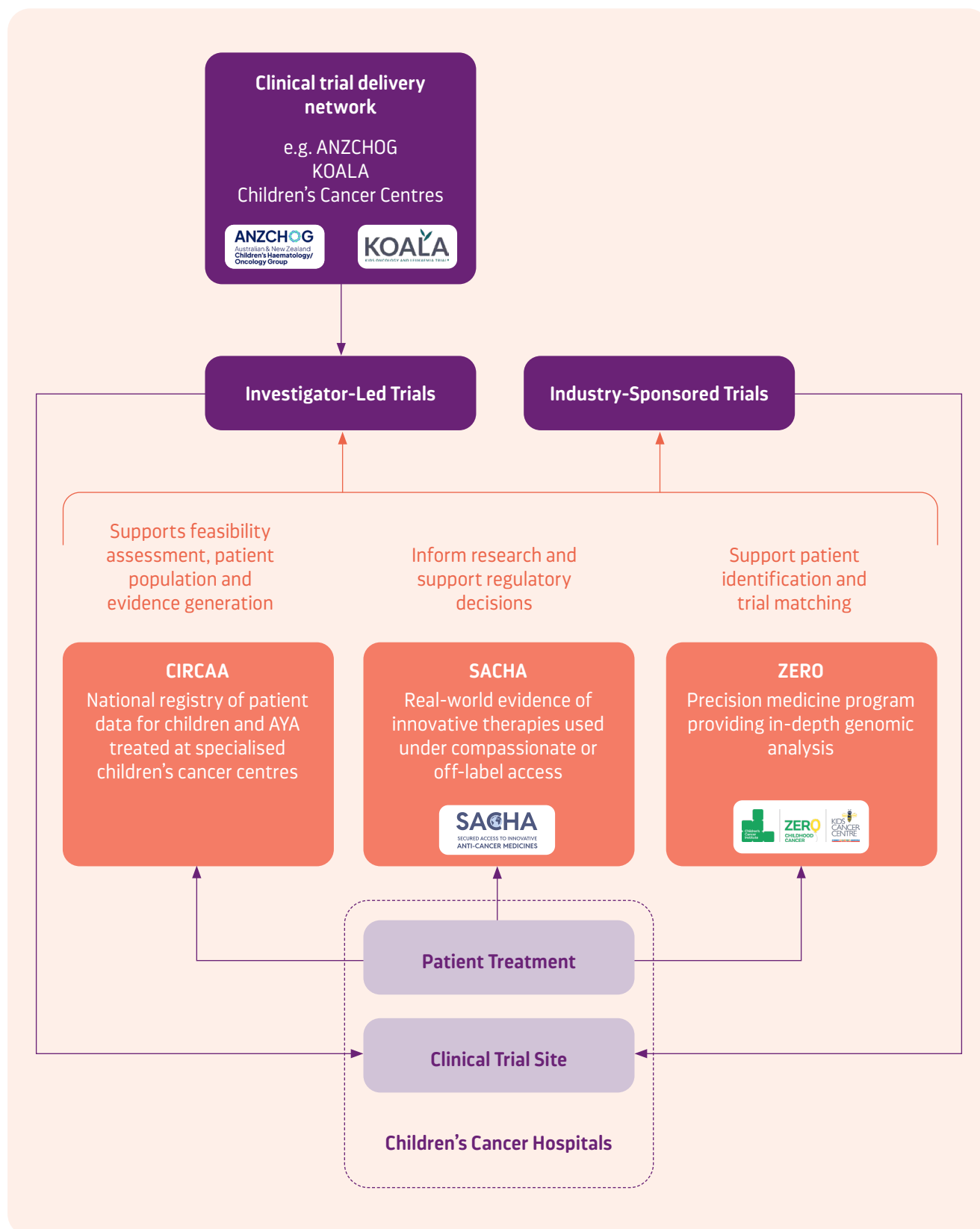
The **Health Technology Assessment (HTA)** process and reimbursement were identified by participants as key areas where Australia is perceived to confer a disadvantage, compared to other comparable nations. Challenges include long timelines from TGA approval to PBS listing. The recent HTA review recommended that PBS listings be age-agnostic where appropriate, giving consideration to patient safety.



Australian researchers and networks enable access to trials

Australia's paediatric oncology ecosystem is built through a network of collaborating organisations and programs that work to support clinical trial delivery. Paediatric cancer treatment is coordinated through specialised children's cancer centres, primarily in each state's capital city. This centralisation of care enables national coordination and interfacing with national programs and initiatives.

Figure 4: Programs supporting Australia's paediatric clinical trial ecosystem



Australian and New Zealand Children's Haematology/Oncology Group (ANZCHOG)



ANZCHOG is the peak professional body and national cooperative clinical trials group that enables access for Australian children with cancer to world-class care. ANZCHOG has well established links with major international clinical trial consortia, such as COG, ITCC, I-BFM, PNOC, CONNECT and others. ANZCHOG acts as the Australian sponsor and coordinating centre for national and international childhood cancer trials, securing and managing funding from public and philanthropic sources.

As the peak professional body for childhood cancer care clinicians, ANZCHOG coordinates national expert groups in all childhood cancer types and areas of care, which play a key role in reviewing and prioritising clinical trial opportunities for Australian children. Membership spans medical, nursing and allied health clinicians, clinical trial professionals and researchers (clinical and scientific).

ANZCHOG also operates the Cancer Information Registry for Children and Adolescents in Australia (CIRCAA), a national registry to record information about paediatric and young adult patients being cared for at Australia's specialist cancer centres where care is primarily centralised.

Kids Oncology and Leukaemia Alliance (KOALA)



The Kids Oncology and Leukaemia Alliance (KOALA) trials at Sydney Children's Hospital was developed to support early phase clinical trials and collaborate with paediatric oncology centres to ensure correct trial implementation. KOALA collaborates with international experts to facilitate the delivery of cutting-edge medical treatment at hospitals around Australia and New Zealand and provides centralised management and coordination of paediatric oncology research.

ZERO Childhood Cancer (ZERO)



ZERO is Australia's national precision medicine program, led by the Children's Cancer Institute at Minderoo Children's Comprehensive Cancer Centre. Working with all nine children's hospitals across Australia, alongside a network of national and international research partners, ZERO provides comprehensive multiomics analysis of each patient's cancer to help identify targeted and individualised treatment options.

Established in 2015, ZERO began as a pilot program before expanding nationally in 2017 to those with high risk, and shortly thereafter to rare and undiagnosable cancers. In 2023, ZERO became available to every child with cancer in Australia, regardless of cancer type or risk profile, and since late 2025, is preparing to expand to young people aged 19–25 with paediatric-type cancers. Close to 4,000 children and young people have now been enrolled, and more children are alive today because of ZERO.

ZERO strengthens Australia's clinical trial ecosystem by identifying patients who may be eligible for targeted and biomarker-driven therapies. This can support more efficient patient recruitment, help match patients to appropriate trials and enable earlier engagement with industry sponsors, making Australia an attractive environment for paediatric oncology research and clinical trials.

Secured Access to Innovative Medicines for Children, Adolescents and Young Adults with Cancer (SACHA)



SACHA is an international registry that records real-world evidence of safety and efficacy of innovative therapies used under compassionate access or off-label access to inform future research and regulatory decisions. The aim of SACHA is to secure access to innovative oncology medicines for children, adolescents and young people outside of the clinical setting.

Case Study: Emily's Story

At just two years old, Emily was diagnosed with a rare childhood brain tumour following a rapid onset of neurological symptoms. Initial treatments included multiple surgeries which eventually successfully removed the tumour. After surgery, concerns of disease recurrence led to clinician recommendation for proton radiotherapy, a treatment only available in the United States. Due to Emily's age, radiotherapy carried significant risk of long-term impacts on brain development.

Through the ZERO Childhood Cancer Program, Emily's tumour was identified as a rarer subtype than initially understood. Evidence suggests that recurrence tends to be in the same location, rather than spread. This additional genomic information enabled clinicians and Emily's family to make the decision to defer radiotherapy and instead pursue close monitoring.

"For everything ZERO has done for our Emily – preserving her crucial brain development for those extra few months by giving her the correct diagnosis, we can't thank you enough," says Sarah.

Several months later, the tumour recurred as predicted in the original location and Emily underwent further surgery at an older age and could proceed with proton radiotherapy with the extra time helping to preserve critical early brain development.

Emily's experience demonstrates the value of precision medicine initiatives such as ZERO in generating tumour-specific evidence that can inform clinical decision-making beyond standard diagnostic approaches.



Challenges for equitable access include geography, variation in awareness of trials and capabilities across states, territories and regional areas

Australia's paediatric clinical trial ecosystem is multifaceted. There are a number of key players, including patients and their families, clinicians, researchers, healthcare services, industry sponsors and governments. The factors outlined in the remainder of this section describe the current state of the paediatric oncology landscape, highlighting the barriers that must be overcome and opportunities that may be leveraged to enable greater trial participation. A summary of participants' perspectives on the current state of the paediatric oncology clinical trials ecosystem is provided in Table 2 below.



Geography, family burden and patient access

Willingness for patients to enrol in clinical trials is heavily influenced by geographic access, financial circumstances, access to support systems and health literacy. While families are often willing to travel and make sacrifices to access trial opportunities, personal circumstances can create challenges such as other caring responsibilities, time away from work and travel. Social determinants of health significantly influence patients' access to clinical trials.

Health literacy also plays a significant role in patients' awareness of clinical trials and willingness to participate. There is a clear need for trusted sources of information, targeted at parents and caregivers, to understand clinical trial opportunities and make informed treatment decisions. While some information exists, patient advocacy organisations indicated that patients and their families are often left to navigate complex clinician-targeted sources of information to understand the options that may be available.



Reliance on researchers and clinicians to drive sponsor engagement

Patients and families are typically made aware of clinical trial opportunities by their treating clinician(s). Variability in the awareness of available clinical trials or the suitability of particular patients to participate may lead to discrepancies in these opportunities being discussed with patients and families. This in turn may impact on clinical trial recruitment and participation.

Researchers and clinicians work collaboratively through established networks and programs, including ZERO, to support sharing of genomic information. Many clinicians and researchers have established relationships with global pharmaceutical companies. They have strong international reputations that support the inclusion of Australian sites in global clinical trials and facilitate compassionate access to emerging therapies. Together and with greater data sharing, these form a strong foundation from which to engage more systematically with industry sponsors.



Variations in capabilities across jurisdictions

While Australia as a whole has strong research and scientific capabilities, there is variation between states and territories in key areas. Clinical trials require fit-for-purpose infrastructure and trained staff. Some states provide dedicated support, while others rely on sponsors or short-term grant funding for contract positions.

There may be opportunities to broaden access to trials across Australian regions using innovative models, such as decentralised trials. Geographical equity is important, given the role that clinical trials may play for some patients in accessing care.

Table 2: Current state of Australia's paediatric oncology clinical trials ecosystem

Enablers 	Barriers 
Clinical and scientific capability: Australia has a strong overall performance in clinical trials, led by cooperative research groups including ANZCHOG that provide ready collaborative infrastructure.	International perception: Lack of awareness of ZERO / trial pathways and the assumption that Australia's relatively small population makes recruitment difficult. Decision-making tends to be US/EU-centric
Precision medicine via ZERO Childhood Cancer Program (ZERO): ZERO supports rapid patient screening and identification at the national level, strengthening Australia's attractiveness to clinical trial sponsors	Most favoured nation pricing: Proposed policies linking US medicine prices to those paid in other countries is creating uncertainty about incentives for pharmaceutical investment outside the US.
Trial start-up framework & incentives: While the CTN scheme supports rapid trial start-up, the R&D Tax Incentive has limited relevance due to multinational paediatric oncology programs	Reimbursement and market access uncertainty: without foreseeable reimbursement pathways, sponsors are deprioritising Australia for trials. PBAC evidence expectations are difficult to meet in small populations.
Centralised paediatric oncology care creates established referral pathways and supports access to trial opportunities through specialist treatment centres	Infrastructure, funding and data: inequity in state-based trial infrastructure and funding. Data access is slow and difficult to integrate between sources.
Clinician-led trial discussions, supported by strong patient awareness and teletrial models, can improve trial participation and extend access beyond metropolitan centres.	Trial design and eligibility: Adult trial age cut-offs (typically ≥18) exclude adolescents who would biologically benefit, particularly for tumours present in both age groups (sarcoma, melanoma, thyroid, carcinoma).
Agile and mobilised community of patients, families and stakeholders	Social determinants of health: Financial, geographic and family burdens can limit trial participation, with clinician knowledge and guidance playing a critical role in connecting patients to appropriate opportunities.

National consortium to align stakeholders and unlock opportunities for industry engagement

1



Create a nationally coordinated consortium to align investment, advocacy and sponsor engagement activities across Australia's paediatric oncology ecosystem

There should be national coordination of efforts to attract paediatric oncology trials

The paediatric oncology landscape in Australia is highly regarded, supported by established research networks, precision medicine programs and clinical infrastructure. However, the ecosystem is complex and not coordinated in such a way that it presents a nationally aligned entry point for industry sponsors. There is a need for coordination on a national level that aligns the interests of international sponsors and Australian stakeholders and communicates Australia's capabilities clearly to global decision-makers.

"Australia is a good place for clinical trials, but [companies are] not really aware of our capabilities, strengths, especially in paediatrics."

While Australia possesses many strong assets, programs and initiatives that operate effectively, these are fragmented across institutions and jurisdictions. This poses a challenge for external stakeholders that may benefit from these resources. As a result, international awareness of Australia's full capabilities in paediatric oncology clinical trials remains limited.

Industry engagement is often dependent on established relationships with individual clinicians, researchers and/or partnerships or technology transfer teams. In the absence of these connections, sponsors may have limited visibility of Australia's capabilities and opportunities. This can deprioritise Australia as a clinical trial destination. Where clinicians and researchers pursue trials independently, it can also place a significant burden on these groups to coordinate engagement and support on an ad hoc basis.

Figure 5: Priority areas for coordination aligned with the Childhood, Adolescent and Young Adult Cancer Roadmap 2026¹⁶

Key Areas of Coordination for a National "Team Australia" Consortium



Research Priorities
Align research around unmet need



Investment
Coordinate funding and infrastructure investment



Stakeholder Engagement
Deliver a unified external voice



Clinical Trial Priorities
Prioritise high-impact trial opportunities



Data
Harmonise data and evidence generation

National coordination is needed to reduce fragmentation and improve system performance

Establishing a nationally coordinated “Team Australia” approach through a consortium of clinicians, researchers, patient organisations, industry and government would facilitate improved outcomes for patients and a more efficient paediatric clinical trial ecosystem.

Australia already benefits from established national clinical trial networks that provide a strong foundation for further coordination. By leveraging existing networks and broadening stakeholder participation, there is an opportunity to enhance international engagement with the goal of attracting more global, industry-sponsored clinical trials to Australia.

While the roundtable discussion did not align on a defined leadership structure for this consortium, organisations such as ANZCHOG already play an important role in national coordination and may be well placed to help convene and facilitate these discussions. By aligning stakeholders around shared priorities, the consortium could assist to focus investments for greatest patient benefit, reduce duplication of effort and maximise the impact of existing clinical, research and advocacy activities. A unified approach could also develop materials and packages of resources targeted to international sponsors to raise awareness and visibility. The nationally coordinated approach should:

- Identify and communicate priority areas of unmet need and strategic opportunities.
- Conduct horizon scanning to identify emerging therapeutic and research opportunities.
- Develop and maintain an outward-facing narrative that clearly articulates Australia’s paediatric oncology capabilities, infrastructure and collaborative networks.
- Create a clear and trusted point of entry for industry sponsors, ensuring information reaches global decision-makers responsible for trial placement and investment decisions.

This coordinated national approach would strengthen Australia’s attractiveness as a destination for industry-sponsored paediatric oncology trials, optimise work already being done in Australia and improve conditions for collaboration.



Develop a pathway for collaboration between industry and investigator-led clinical trial models

Australia has a strong track record of investigator-led paediatric oncology trials that represent valuable knowledge and resources within the ecosystem. These capabilities include valuable clinical expertise, established trial infrastructure and support for identifying emerging areas of need which can be leveraged by industry sponsors to accelerate trial implementation.

Fostering collaboration between industry and established researchers is mutually beneficial. Roundtable participants confirmed that investigators contribute deep knowledge of the Australian clinical trials ecosystem, while industry brings innovative medicines, a pathway to market and investment. A consortium-based approach would provide a forum to establish a practical pathway for increasing the attraction of industry-sponsored paediatric oncology trials to Australia.

Local industry representatives play a critical role in translating the outputs of Australia's coordinated paediatric oncology ecosystem to global decision-makers responsible for trial design, investment and trial placement. Given that limited international visibility is frequently a barrier to attracting trials, a collaborative approach enables visibility of opportunities within Australia that may be mutually beneficial and improve access to clinical trials for Australian patients.

Table 3: Recommendations to establish Team Australia approach to clinical trials

Actions	Industry	Clinicians, Researchers and MRIs	State Government	Federal Government
Hold an annual forum of the Australian ecosystem to engage with leading global trial sponsors in paediatric oncology to understand needs and share information about best practice and Australian case studies				
Consortium based coordinated approach to advocacy				
Coordination of approach to reach out to global sponsors, prioritising areas of unmet need (as identified through Child and AYA Cancer Roadmap and other activities)				
Advocate for Australian trial sites within global organisations, focusing on the key competitive advantages of Australia's ecosystem to attract industry-sponsored trials				

LEAD SUPPORT

Integrated data infrastructure to strengthen evidence generation and clinical utility

2



Build on existing data infrastructure to enhance the utilisation of data and create a more connected and collaborative ecosystem

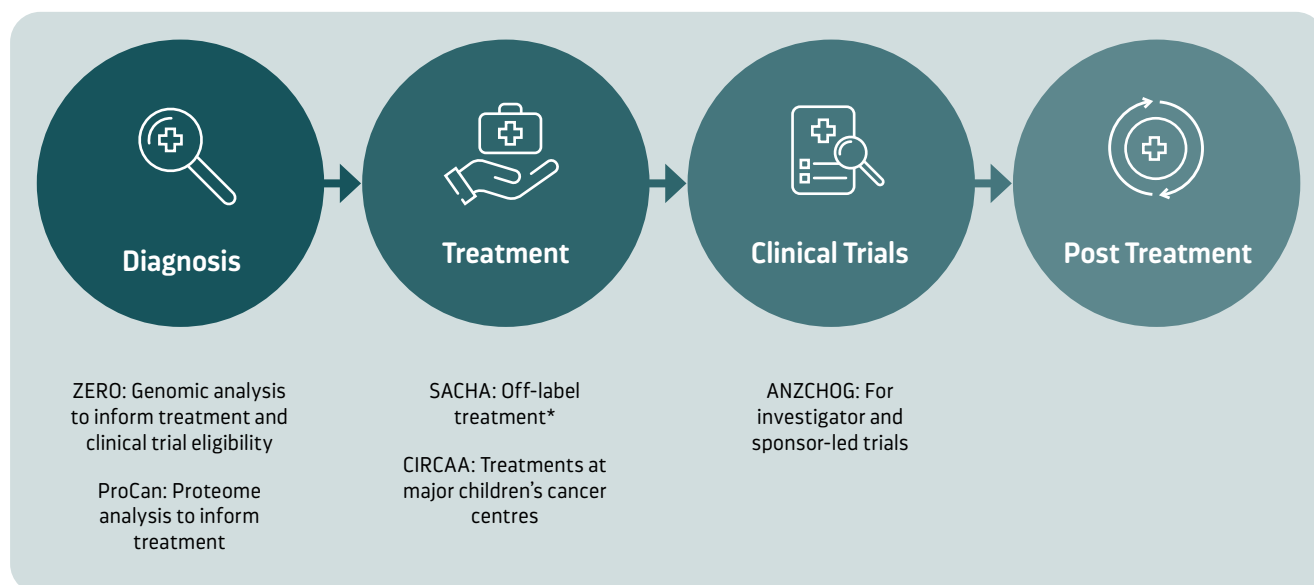
Australia's advanced paediatric oncology data remains fragmented, limiting its value for clinical trials and evidence generation

Australia has some of the most advanced data records in the world for children with cancer, but they remain fragmented across various systems, with limited continuity constraining their value to the paediatric clinical trials ecosystem. Programs such as ZERO, CIRCAA and SACHA, together with broader infrastructure such as OMICO, each perform important functions that record critical aspects of a patient's treatment journey. However, these data do not track a patient's treatment journey from diagnosis to long-term outcomes.

"We sit on an enormous asset... but we don't know anything"

Greater integration and accessibility could allow paediatric oncology data to support real-time clinical decision-making, trial identification and recruitment, and sponsor-led generation of real-world evidence. The primary limitation in integrating these systems is the inconsistency that arises from various governance arrangements (including data access), database formatting and patient identification across various intake systems. Rather than creating new systems, the priority is to build on existing infrastructure and enable greater utilisation of available data.

Figure 6: Patient journey and data collection *in addition to standard of care diagnostics and treatment protocols*



*No SACHA site has yet opened in Australia, and the program lacks adequate funding.

Paediatric oncology already has a robust foundation for national data linkage, supported by existing infrastructure

Paediatric cancer care is predominantly centralised within specialised children's hospitals in Australia's major capital cities. These hospitals have existing data arrangements with ZERO, providing precedent for a unified process of data sharing across institutions. Compared with the challenges of implementing a broader state or country-wide system, paediatric oncology represents a smaller and contained area of the health system within which adoption of data linkage processes can be driven by the few, specialised children's cancer centres.

“ZERO works because we've all been working together, and that's the most powerful”

Connect existing assets to improve utilisation

Efforts should be made to link data from existing assets such as ZERO, CIRCAA and SACHA in a way that is accessible to researchers and clinicians for informed decision-making in patient treatment and trial design¹⁷. This includes establishing technical standards and processes to link data across the patient journey and identify gaps that existing programs do not address. Relevant information should be made available in real time for clinicians to identify potential treatment options and refer patients to appropriate clinical trials.

In addition to the data that is already recorded, clinicians and patient advocate participants at the roundtable indicated that many families want and expect their experiences to contribute to learning and improving outcomes for future children. This reflects a willingness among patients and families to provide informed consent for data sharing.

Roundtable participants whose roles related to data governance indicated potential concerns about the handling and sharing of data across some scenarios. For example, for compassionate access programs, companies do not collect data and use consent forms that expressly prohibit the use of data for future regulatory purposes. Clear governance structures are required to enable the use of data in a way that is mutually beneficial for patients, clinicians and clinical trial sponsors.

Detailed consideration of the governance structures required to support data linkage and sharing is required. General views expressed at the roundtable support the Child and AYA Cancer Roadmap's objective to “reduce duplication by strengthening centralised data collection and coordinated management of child and AYA data across registries, clinical systems and research platforms” (RCT3.2)¹⁸.

Establish governance, consent and access arrangements

Nationally consistent governance, consent and access arrangements should be established to remove uncertainty about how data is allowed to be used. These arrangements should clearly detail responsibilities, ownership, access rights, approved uses and custodianship as well as security protections that are required of stakeholders across the paediatric clinical trials ecosystem.

Arrangements should incorporate a clear roadmap for how data can be utilised and ensure authorisation for data to be used to improve outcomes for future children with cancer. Roundtable participants felt that consent processes should enable patients and families who wish to contribute their data to do so, while providing researchers and sponsors with the clarity and confidence to use that information appropriately for research, regulatory and reimbursement purposes that may improve access to innovative therapies.

Strengthen evidence generation to inform trial design and support regulatory submission

Industry, researchers and healthcare providers should work together to consistently collect treatment and outcomes data through clinical trials and compassionate access programs. Given the rarity of childhood cancers, paediatric trials often recruit small patient populations, which can limit their ability to generate sufficient evidence against intended trial outcomes. Where possible, real-world data and long-term outcomes should be linked with existing national datasets to consider alongside clinical trial findings where additional evidence could help address uncertainty and support future regulatory, reimbursement or policy decision-making.

Table 4: Recommendations to optimise utilisation of data infrastructure

Actions	Industry	Clinicians, Researchers and MRIs	State Government	Federal Government
Support efforts identified in the Child and AYA Cancer Roadmap to “reduce duplication by strengthening centralised data collection and coordinated management of child and AYA data across registries, clinical systems and research platforms” (RCT3.2)				
Establish appropriate governance arrangements and technical systems to make data available in real-time to support clinician decision-making and potential referrals to clinical trials, to improve clinical trial recruitment				
Integration of the data ecosystem that links to existing assets e.g. ZERO, CIRCAA, SACHA				

LEAD SUPPORT

Equitable access through decentralised trial delivery

3



Expand equitable access through decentralised trial delivery and strengthened site capabilities

Uneven access to trials is constraining both patient outcomes and recruitment potential

Despite a strong investigator-led paediatric cancer clinical trial network, access to trials is not consistent for all patients with factors such as geography and family financial circumstances playing a significant role in treatment options. Access may also be affected by variation in clinical trial infrastructure, funding arrangements and workforce (by state and across regional centres). As a result, opportunities to participate in clinical trials and access innovative therapies are not equitably distributed across Australia. This variability also limits Australia's attractiveness as a clinical trial destination due to the challenges in consistently reaching and recruiting patients where they live.



Reducing participation barriers benefits families, sponsors and the broader research ecosystem

Optimising patient access to clinical trials delivers two key benefits:

1. Ensures that all children and adolescents have the opportunity to participate in research and access innovative treatment, regardless of location or socioeconomic circumstances.
2. Increases the pool of patients available for recruitment, thus improving enrolment rates and making Australia a more attractive destination for clinical trials.

Advances in telehealth, decentralised trial delivery and shared-care models may help to alleviate some geographical barriers to participation. Access to routine monitoring, follow-up care and other appropriate aspects of care could be expanded beyond major metropolitan centres. This would lower the burden on children and families to relocate for the purpose of accessing a trial and minimise disruption of their lives. Patient advocates play an important role in the design of appropriate telehealth services that can meet the needs of and improve the experiences of patients and families. Equitable access standards would help to ensure that clinical trial participation is not hindered by a patient's personal circumstances.

“Families will travel. They will do what they need to do for their children, but there is a [burden] with that, and it does make accessing those populations a lot harder.”

Build a national trial delivery network that reaches patients wherever they are

ANZCHOG has developed a formal decentralised trial framework that is significantly different to the adult model. It provides structure for the implementation of teletrial elements where safe and feasible, to improve equity of access, noting that a key difference between childhood and adult cancer care is the lack of appropriate clinical expertise to care for children with cancer in most regional centres. It is not realistic to try to implement adult teletrial models, but the principles allow for access to limited site trials for children and young people cared for in other specialist centres around the country.

Patient advocates play a critical role in supporting the design of trials that enrol patients outside of major centres. The care provided must be equivalent to that provided in specialist services for patients and families to participate. Ongoing engagement with patient advocates can both improve the design of the service and ensure that the needs and experiences of patients and families are appropriately addressed.

“Adults have actually shown you do trials utilising telehealth, that would provide the equity and shared care opportunities [we're aiming for].”

Case Study: Decentralised Clinical Trials in Australia

To address barriers presented by Australia's geographic spread of patients, Servier developed and piloted a first-in-human adult oncology study using its Rapid Activation Trial Model (RAD-TM). The model was designed to improve patient access and reduce participation burden while strengthening recruitment and operational efficiency through a national comprehensive genomic screening program and a decentralised trial-delivery model.

The initiative was both patient- and sponsor-driven, combining the need to improve equitable access for geographically dispersed patients, improve disease knowledge through comprehensive genomics, integrate Trusted Care into study design to keep participants connected with their local health support, and obtain faster answers for the study by accelerating recruitment. Its success was enabled by a unique collaboration of industry partners working together to translate a decentralised model into practice.

Successful implementation of the RAD-TM model relied on three critical components: national patient identification, hub-and-spoke delivery, and rapid activation of regional satellite sites. Patient identification and referral was supported through OMICO, the adult equivalent of the ZERO precision medicine program.

Rather than relying on a fully centralised model, patients were initially managed through specialised metropolitan phase 1 centres, which were primarily responsible for patient care and initial treatment. Once patients were stable, full trial participation could be delivered closer to home through regional satellite sites, enabling ongoing participation without additional travel requirements.

Regional hospitals could be trained as research sites with local clinicians, supported by metropolitan investigators. This hybrid approach reduced the need for regional sites to operate entirely independently in challenging early phase studies, providing both patients and clinicians with flexibility and confidence.

With the RAD-TM model in place, Australia became the highest recruiting country in the study globally, contributing 37% of total global recruitment and being 3.5 times more productive than comparable international sites. For patients, access was expanded, with 35% of patients pre-screened referrals from regional or rural areas.

While implemented in an adult oncology setting, the model was created for agile deployment and scalability across diseases and trial phases, with features relevant to the recruitment challenges of rare and hard-to-treat diseases, including paediatric oncology studies targeting biomarkers. This highlights how existing ecosystem assets can be coordinated to expand patient access and improve trial recruitment performance.

Kong JB, Cockburn C, Ameratunga M, McPhee N, Harris S, Rubio-Jareno A, Denis G, Ferreira D, Woollett A, Evans W, Shackleton K, Thomas D, Sebastian L, Lemech C, Talarico A, Zielinski R, Stewart J, Tusler M, Robinson C, Hinds F. Advancing Equity Through Collaboration: A Patient-Centric Decentralised Model for First-in-Human (FIH) Studies. ESMO Congress 2026 Abstract. ESMO Congress 2026: Abstracts. European Society for Medical Oncology. Available at: ESMO Congress 2026: Abstracts.



Create a Rapid Trial Activation Framework for Paediatric Oncology

Establishing standardised trial activation protocols is a key tool to increase Australia’s attractiveness as a clinical trial destination in paediatric oncology where there may be only a small number of eligible patients, often distributed across multiple jurisdictions and care settings. By minimising delays associated with patient enrolment, sponsors are able to improve trial accessibility and recruitment performance. This also creates the potential for decentralised delivery models, such as Servier’s Rapid Activation Trial Model (RAD-TM). Australia could establish a coordinated trial delivery package that streamlines trial start-up and presents capabilities to sponsors to strengthen Australia’s competitiveness as a trial destination.

Table 5: Recommendations for equitable access to clinical trials

Actions	Industry	Clinicians, Researchers and MRIs	State Government	Federal Government
Develop minimum standards for access to teletrials, hub and spoke and other approaches to decentralised trials across states and territories				
Leverage regional healthcare networks, telehealth and shared-care models to support appropriate trial-related activities, reducing participation burden while maintaining specialist oversight.				

 LEAD  SUPPORT



A clearer policy and commercial pathway can improve Australia's competitiveness

4



Align commercial incentives with the realities of paediatric oncology development, including regulatory and reimbursement frameworks to provide a pathway to market following clinical trials.

Australia's policy setting does not adequately support commercial realities of paediatric oncology trials

A company's decision to conduct a clinical trial in Australia is influenced by commercial realities. Industry participants indicated that they are more likely to include Australian sites in global development programs when there is confidence that evidence generated through paediatric studies can support timely regulatory approval, reimbursement and patient access. Clinicians also stated a preference for clinical trials over compassionate access, as it contributes to the generation of evidence that can support future regulatory approval, reimbursement and broader patient access.

Paediatric oncology faces many of the same evidence generation challenges as rare diseases; however, access processes still rely on conventional evidence standards. These are difficult to satisfy for paediatric oncology, where indications are generally designated as rare diseases. Although real-world evidence may exist to alleviate some of this uncertainty, industry participants indicated that it can be unclear how these sources are considered and whether they influence decision-making in regulatory and reimbursement assessments.

In some cases, paediatric oncology therapies that qualify for an orphan drug designation can access regulatory benefits, including waived fees for TGA registration and the first PBAC submission. The practical value of the latter incentive may be limited since 87% of rare disease drugs require more than one submission before receiving a positive recommendation¹⁹. The need for multiple submissions extends market access timelines even when therapies are eligible for fast-tracked TGA consideration for serious and life-threatening conditions.

Clarifying the requirements for accepting real-world evidence and considering managed access or coverage with evidence development pathways could support the commercial case in Australia for paediatric oncology medicines. Addressing the commercial realities was considered important for roundtable participants.

"Some companies are making decisions based on that. If they don't see a pathway to reimbursement, they won't [prioritise an Australian trial site]."

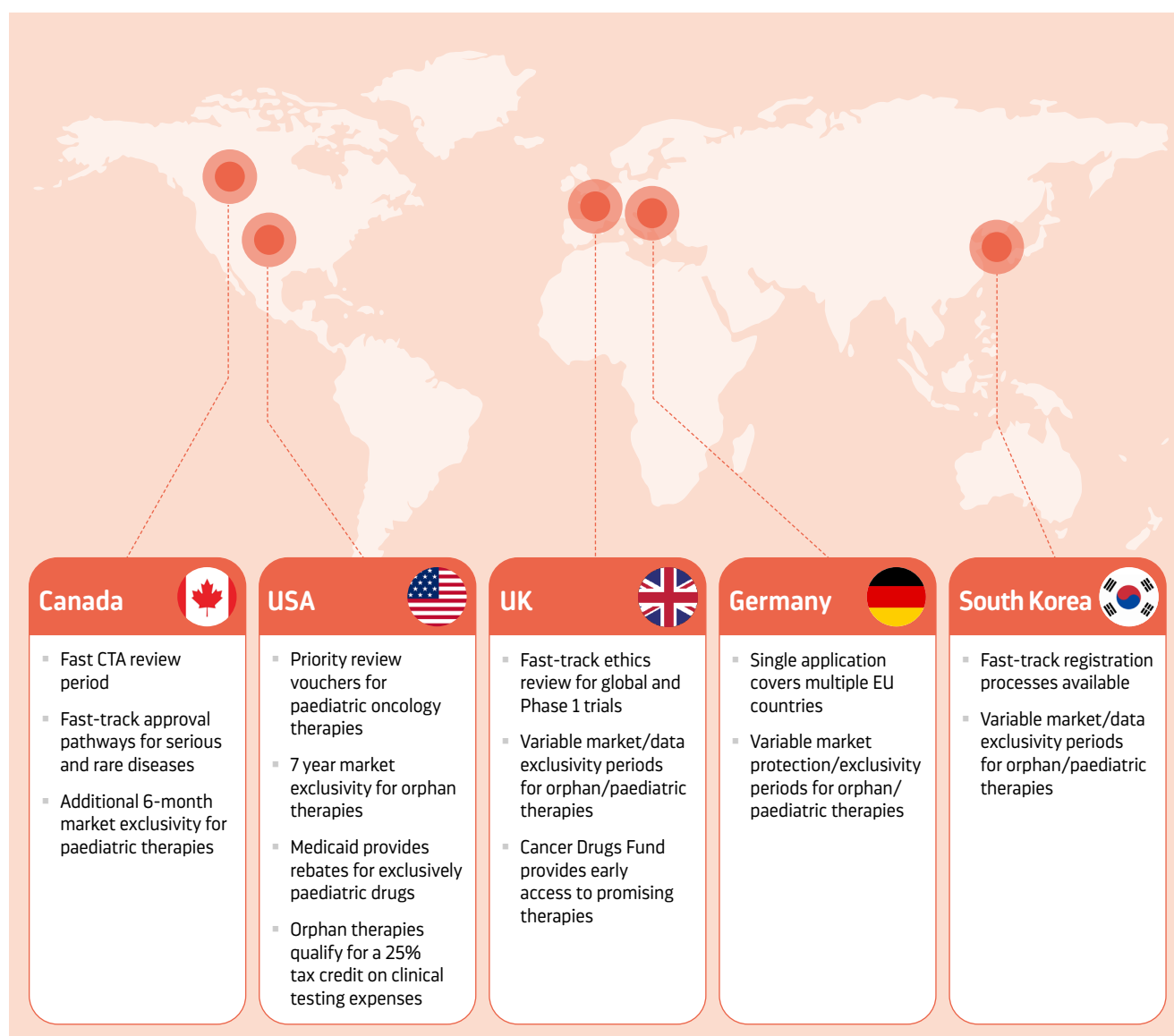
Alignment with international best practice creates a pathway for commercial value that increases attractiveness of Australia's offering

Paediatric medicines are typically designated as orphan drugs by international regulators, which confers special considerations through regulatory and reimbursement processes. These include expedited assessment pathways, market exclusivity provisions and early access pathways.

At present, Australia's policy settings offer incentives for larger therapeutic markets and are not aligned with international approaches to paediatric oncology development. This may explain why Australia outperforms peers in attracting industry-sponsored adult oncology trials, but not for paediatric trials.

National policies like the R&D Tax Incentive (RDTI) may attract early phase trials but are less applicable to later phase paediatric oncology trials which are typically multinational and unlikely to satisfy requirements for research activity being conducted in Australia. International case studies for enabling launches of paediatric medicines include market protection and fast-tracked pathways (Figure 7).

Figure 7: Policies for paediatric oncology regulatory and reimbursement processes in selected countries



Formalise a systematic approach to age-agnostic regulatory and reimbursement pathways

Age restrictions on regulatory and reimbursement approaches can create barriers to access for young people. There is uncertainty regarding the evidence requirements needed to support inclusion of a paediatric population. As a result, sponsors may be reluctant to seek a paediatric indication or age-inclusive reimbursement submission, particularly in settings where patient numbers are low and evidence may be immature.

Where possible, giving appropriate consideration to patient safety, regulatory and reimbursement frameworks should accommodate the unique challenges for paediatric oncology. Australia's PBAC has been shown to offer greater flexibility in approving paediatric medicines. This could be formalised. Adopting Recommendation 2 of the Health Technology Assessment Policy and Methods Review would prioritise age-agnostic PBS listings.

Clarify the role of real-world evidence in paediatric regulatory and reimbursement approvals

Paediatric oncology indications are typically classified as rare diseases, which makes evidence generation challenging. It can be difficult for sponsors to gather a standard evidence package that meets the requirements of regulatory and reimbursement decision-making. Roundtable participants identified real-world evidence as a potential mechanism for addressing this uncertainty; however, they noted a lack of clarity regarding how this data is considered by the TGA and PBAC.

Greater acceptance of real-world evidence was identified as an opportunity to provide certainty around the commercial opportunity for sponsors aiming to bring paediatric therapies to Australia. This could then position Australia as a more attractive market to invest in, in the competitive international context. Sponsors may also invest more in Australian data resources, such as patient registries, and observational studies, including longitudinal outcomes. The overall effect could increase the evidence base for paediatric therapies.

Table 6: Recommendations for increasing the commercial attractiveness of Australia as a clinical trial destination

Actions	Industry	Clinicians, Researchers and MRIs	State Government	Federal Government
Review Australia's overall competitive positioning to attract global trials, introducing taxation and subsidies, orphan drug policies for regulatory and reimbursement processes and other mechanisms used to attract trials internationally (with a specific focus on paediatric oncology)				
Implement recommendation 2 of the HTA Review Report: To formalise a systematic approach for new PBS listings to be age-agnostic, and to review existing listings for potential removal of age restrictions				
Review current barriers to regulatory approvals through age restrictions, and potential for age agnostic approvals (where scientifically justified)				
Formalise approaches to acceptance of real-world evidence that would enable regulatory (ARTG) and reimbursement (PBS) listings in paediatric age groups where there is uncertainty in clinical trial data				

 LEAD  SUPPORT

Concluding statements

Children with cancer face many unique challenges to access innovative treatments, due to the rare and complex nature of their disease. Clinical trials offer a critical access pathway to new and emerging treatments.

The National Paediatric Oncology Industry-sponsored Clinical Trial Roundtable highlighted Australia's significant strengths, especially in research and clinical expertise and infrastructure, that could be leveraged to expand opportunities for children with cancer to access clinical trials. Stakeholder support, policy reforms and investments are required. By bringing together leaders from industry, research, clinical care, government and the patient community, the roundtable identified key recommendations to strengthen industry engagement with the clinical trial ecosystem.

In an increasingly competitive international clinical trial landscape, there is a need for ongoing review, decisive action, and commitment to continuous improvement to remain globally competitive. Meaningful change will require a coordinated, sector-wide effort with responsibility shared across industry, researchers, clinicians, government, healthcare services and the patient community rather than a single organisation. Building on existing strengths and addressing barriers with a mobilised stakeholder community, we can continue to offer expanded trial opportunities for Australian children with cancer.



Appendix

Acronym	Full term
ANZCHOG	Australian and New Zealand Children's Haematology/Oncology Group
ARTG	Australian Register of Therapeutic Goods
AYA	Adolescent and Young Adult
CCI	Children's Cancer Institute
CIRCAA	Cancer Information Registry for Children and Adolescents in Australia
CTN	Clinical Trial Notification
HREC	Human Research Ethics Committee
HTA	Health Technology Assessment
KOALA	Kids Oncology and Leukaemia Alliance
MA	Medicines Australia
MRI	Medical Research Institute
NOSS	National One Stop Shop
OMICO	Australian Genomic Cancer Medicine Centre (OMICO)
PBAC	Pharmaceutical Benefits Advisory Committee
PBS	Pharmaceutical Benefits Scheme
RAD-TM	Rapid Activation Trial Model
RDTI	Research and Development Tax Incentive
SACHA	Secured Access to Innovative Medicines for Children, Adolescents and Young Adults with Cancer
TGA	Therapeutic Goods Administration
ZERO	Zero Childhood Cancer Program

Endnotes

- 1 “About Childhood Cancer,” Zero Childhood Cancer, accessed August 24, 2026, <https://www.zerochildhoodcancer.org.au/about/about-childhood-cancer>.
- 2 Zero Childhood Cancer, “About Childhood Cancer.”
- 3 *Disparities in Potentially Preventable Hospitalisations across Australia, 2012-13 to 2017-18* (Australian Institute of Health and Welfare, 2019).
- 4 Danny R. Youlden et al., “Childhood Cancer Survival and Avoided Deaths in Australia, 1983–2016,” *Paediatric and Perinatal Epidemiology* 37, no. 1 (2023): 81–91, <https://doi.org/10.1111/ppe.12895>.
- 5 M. Angeles Vázquez López, “Childhood Cancer Survivors: Current Challenges,” *Anales de Pediatría (English Edition)* 100, no. 5 (2024): 363–75, <https://doi.org/10.1016/j.anpede.2024.04.013>.
- 6 Margit K. Mikkelsen et al., “The Global Clinical Trial Landscape for Children and Adolescents With Cancer,” *JAMA Network Open* 9, no. 1 (2026): e2552510–e2552510, <https://doi.org/10.1001/jamanetworkopen.2025.52510>.
- 7 “ANZCTR,” accessed August 24, 2026, <https://www.anzctr.org.au/Default.aspx>.
- 8 GlobalData Healthcare, “GlobalData Clinical Trials Database Search: Interventional Paediatric Oncology Trials in Australia,” Clinical trials database, n.d.
- 9 Mikkelsen et al., “The Global Clinical Trial Landscape for Children and Adolescents With Cancer.”
- 10 “WHO Landscape of Clinical Trials on Cancer,” accessed August 24, 2026, <https://www.who.int/observatories/global-observatory-on-health-research-and-development/monitoring/clinical-trials-on-cancer>.
- 11 MTPConnect, “Australia’s Clinical Trials Sector,” June 2024, <https://auscelerate.org/wp-content/uploads/2025/07/MTPConnect-2024-Australias-Clinical-Trials-Sector-Report.pdf>.
- 12 “New Report Features Australia’s Clinical Trial Advantage,” accessed September 9, 2026, <https://biotechdispatch.com.au/news/new-report-features-australias-clinical-trial-advantage>.
- 13 ANZCHOG, “2024 Annual Review,” 2024, <https://anzchog.org/wp-content/uploads/2025/09/Annual-Review-2024.pdf>.
- 14 GlobalData Healthcare, “GlobalData Clinical Trials Database Search: Interventional Paediatric Oncology Trials in Australia.”
- 15 GlobalData Healthcare, “GlobalData Clinical Trials Database Search: Interventional Paediatric Oncology Trials in Australia.”
- 16 Cancer Australia, “Childhood, Adolescent and Young Adult Cancer Roadmap,” Cancer Australia, May 2026, <https://www.canceraustralia.gov.au/sites/default/files/2026-05/childhood-adolescent-and-young-adult-cancer-roadmap-2026.pdf>.
- 17 Cancer Australia, “Childhood, Adolescent and Young Adult Cancer Roadmap.”
- 18 Cancer Australia, “Childhood, Adolescent and Young Adult Cancer Roadmap.”
- 19 IQVIA, “Access to Orphan Drugs in Australia,” Whitepaper, 2024, <https://www.iqvia.com/-/media/iqvia/pdfs/australia-and-new-zealand/white-papers/access-to-orphan-drugs-in-australia.pdf>.



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