

Annual Review 2024

The logo consists of a green geometric shape made of several squares and rectangles, resembling a stylized building or a cluster of cells.

Children's
Cancer
Institute



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About Children's Cancer Institute

Children's Cancer Institute is the only independent medical research institute in Australia wholly dedicated to research into the causes, prevention and cure of childhood cancer. Our vision is to save the lives of all children with cancer and improve their long-term health, through research. Our focus is on translational research, and we have an integrated team of laboratory researchers and clinician scientists who work together in partnership to discover new treatments which can be progressed from the lab bench to the beds of children on wards in hospitals as quickly as possible. By developing safer and more effective drugs and drug combinations, we aim to minimise side effects, giving children with cancer the best chance of cure with the highest possible quality of life.

Front cover: Jacob was diagnosed with leukaemia at four years old.

Inside front cover: Carys Dawson, diagnosed with leukaemia at six-years-old, with dad Mark, mum Laura and brother Cohle.

It's been a year of great progress

At Children's Cancer Institute, we believe a life should be long. That every child should have the chance to grow up, grow old and live a life full of possibilities. In 2024, our passionate and dedicated researchers continued to push the boundaries of what's possible, to create a brighter future for children with cancer.

2024 was particularly significant for the Institute, because it marked 40 years since our laboratories first opened. We were founded by a small group of parents and doctors of kids with cancer who asked themselves: 'What can we do to make a difference?' And it's people like these, who ask the same question, who continue to push us towards our ultimate goal: a world where no child dies of cancer.

The year was not without its challenges, with the entire medical research sector facing difficulties in terms of sustainability and future growth. The growing gap between the true cost of research and the direct costs funded by government is

a significant issue, with approximately 84% of independent medical research institutes in Australia currently operating at a loss.

To address this sustainability challenge, we underwent an organisational review to ensure that our operations are not only fit for purpose but also as efficient and streamlined as possible for the future. We also implemented a 'true cost of research' framework aimed at both realising direct cost recovery and, wherever possible, recovery of the actual indirect infrastructure costs.


Throughout the year, our research continued to gain momentum. Our team made significant breakthroughs in identifying new treatment options for children with cancer, with a new drug found to yield extraordinary results in T-cell acute lymphoblastic leukaemia, an aggressive leukaemia mainly affecting children, and a whole new approach uncovered to treating neuroblastoma, the most common solid tumour in children under five.

The Zero Childhood Cancer Program (ZERO) – our national precision medicine program for kids with cancer which we co-lead with the Kids Cancer Centre at Sydney Children's Hospital, Randwick – grew substantially, not only in terms of the number of children enrolled, now over 2,500, but also the number of hospitals participating, with two hospitals in New Zealand joining all nine Australian children's hospitals as part of the Program.

In June, the latest results from ZERO were published in Nature Medicine, one of the world's most prestigious medical research journals, showing that the precision medicine approach taken by ZERO significantly improves survival in kids with high-risk cancer. In fact, the 2-year progression-free survival of children who received their ZERO-recommended personalised treatment was more than double that of children who received standard therapy. Put simply, more children are surviving because of ZERO and as a result, the entire model of care for kids with cancer in this country is changing.

And now, after years of planning, we couldn't be more excited about our imminent move to the Minderoo Children's Comprehensive Cancer Centre. We look to the future with great optimism, knowing we will be in the ideal environment to conduct our world-class science and see our findings translated into real-world settings, ultimately leading to improved outcomes for kids with cancer.

Thank you to all of you who have been with us along this journey. Together I believe we can, and we will, cure every child of cancer. As we say at the Institute, it's not if, it's when.



Professor Michelle Haber AM
Executive Director
Children's Cancer Institute



In 2024, Children's Cancer Institute celebrated 40 years since our lab doors first opened.



2024 snapshot



350
staff

177
researchers

39
students



33
awards/honours
received

24
internal research
promotions



827
children enrolled on
ZERO in 2024

>2500
in total



14
UNSW academic
appointments

10
UNSW academic
progressions



119
publications

45%
with our researchers
as first/senior author



12
active clinical trials
supported by our
research

400
collaborators
worldwide



237k
social media
followers

72m
audience
media reach



54,924
donors

37,761
new donors

Financial summary

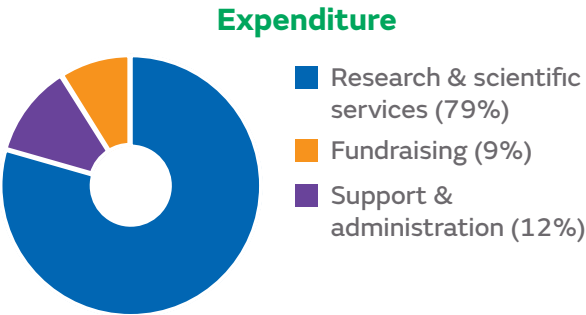
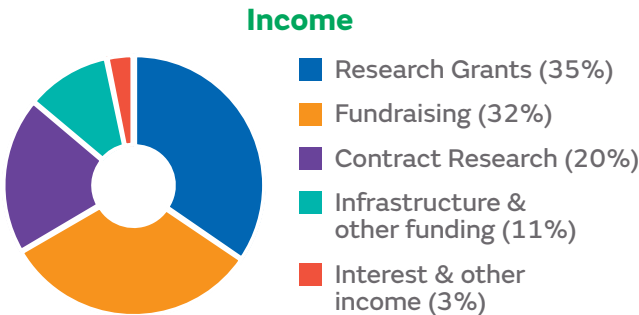
2024 was a stronger financial year than 2023, with growth in income and containment of expenses resulting in a significantly reduced deficit year on year.

Our income grew by 4% due to strong fundraising and competitive research funding results. Our expenditure was contained to below 2023 levels, due to implementation of a range of operational efficiencies during 2024, together with the rollout of more balanced research pricing and costing

policies, and the commitment of both Management and Board to operate at better-than-break-even for our non-research support and admin activities from quarter four. The full benefits of these efficiency changes will continue into 2025 and beyond.

The vast majority (79%) of all funds raised, earned or awarded were again spent directly on employing researchers and conducting research and scientific service activities.

Income	2024	2023	
	\$'000	\$'000	%
Research grants	29,819	28,541	35
Contract research	16,850	21,440	20
Infrastructure and other research funding	9,117	9,177	11
Fundraising	27,657	21,376	32
Interest and other income	2,898	2,399	3
Total	86,341	82,933	
Expenditure	2024	2023	
	\$'000	\$'000	%
Research and scientific services	70,031	69,918	79
Fundraising	7,885	9,172	9
Support and administration	10,249	9,530	12
Total	88,165	88,621	
Surplus (Deficit) for the year	- 1,824	- 5,688	



February

Hope for young Australians with sarcoma

A new initiative to help young Australians fighting sarcoma, known as the 'Paediatric Preclinical Biobank', is made possible through funding of \$312,000 by the Cooper Rice-Brading Foundation, which received a generous donation from the Sutcliffe Family Trust.



March

Hitting the pavement

Our month-long 86k for a Cure community event sees over 7000 supporters hit the pavement to walk, run or do 86 of anything to represent the 86 children diagnosed with cancer every month in Australia. The extraordinary community effort sees over \$2.1m raised for our childhood cancer research.

April

MCCCC reaches major milestone

The Minderoo Children's Comprehensive Cancer Centre (MCCCC) reaches a major milestone, with construction of the new 12-storey building reaching its highest point.



May

Sharing data with the world

Our Computational Biology team build the Zero Childhood Cancer Data Portal – known as BEACON, a purpose-built platform to enable sharing and exploration of the rich datasets collected and generated through ZERO. This is hoped to be a significant step in ZERO's contribution to the global childhood cancer research effort.

June

World-first study yields extraordinary results

Results from the Zero Childhood Cancer Program (ZERO) are published in the prestigious international journal, Nature Medicine, showing that its precision medicine approach significantly improves survival in children with high-risk cancer. The 2-year progression-free survival of children who received their ZERO-recommended personalised treatment was more than double that of children who received standard therapy.

2024 in review

November

Grant to transform outcomes for children

We are awarded a \$5 million grant from the Australian Cancer Research Foundation (ACRF) to establish the groundbreaking ACRF Childhood Cancer Early Detection, Prevention, and Treatment (ACCEPT) Program, aimed at drastically reducing childhood cancer deaths and transforming patient outcomes.

November

New drug yields extraordinary results in childhood leukaemia

Our researchers find that a new-generation drug developed to treat an aggressive leukaemia that mainly affects children, T-cell acute lymphoblastic leukaemia (T-ALL), has 'excellent potency' both as a single agent and in combination with other therapies.

October

Researchers part of 'Top 100 Innovators' list

We are proud to see two of our researchers, Associate Professor Paul Ekert and Chelsea Mayoh, feature in *The Australian* newspaper's 'Top 100 Innovators 2024' list. The researchers made news last year when they published world-first results showing that many more children with cancer than previously realised could potentially benefit from immunotherapy.



September

Our most successful Diamond Ball ever

Our signature Diamond Ball event is held in Sydney, with almost 800 of our most special supporters in attendance, raising \$2.1m for our childhood cancer research — the highest amount ever raised at this event.



August

New research project to combat deadliest childhood cancer

A bold research project that could lead to the first-ever effective drug to treat the deadly brain cancer, diffuse intrinsic pontine glioma (DIPG), receives a major boost with a \$US400,000 grant awarded to Dr Jean Bertoldo by the US-based charity, ChadTough Defeat DIPG Foundation.

July

Potential new approach to neuroblastoma discovered

Research led by Professor Murray Norris AM discovers for the first time that a gene known as RUNX1T1 is essential for the development of neuroblastoma, opening up the possibility of a whole new approach to treating this aggressive childhood cancer.

Pioneering ZERO Childhood Cancer

In June, world-first results from the Zero Childhood Cancer Program (ZERO) were published in the top international journal *Nature Medicine*. These results prove that the precision medicine approach taken by ZERO is superior to standard therapy in children with high-risk cancer, both in terms of clinical response and survival.

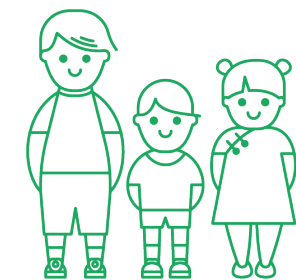
Of the 384 children on the study, children who received a ZERO-recommended treatment did significantly better than those who did not. Their 2-year progression-free survival was more than double that of children who received standard therapy (26% vs 12%), and five times higher than that of children who received an unguided treatment (a novel agent not chosen on the basis of molecular findings).

By showing that outcomes can be radically changed if treatment is tailored to the individual, ZERO is changing national and international clinical practice. More children are surviving cancer, and the entire model of care for children with cancer is changing.

ZERO turns ten

Over the last 10 years, ZERO has gathered incredible momentum. When it was first launched as a pilot program for children with high-risk cancer in 2015, there was no precedent for this ambitious precision medicine program, no medical research institute had ever been able to individualise genomic research for every patient. In 2017, ZERO went national. Then, with support from the Commonwealth Government and Minderoo Foundation, in 2023 it expanded to become available to all Australians aged 0-18 years with cancer, regardless of their risk profile, making Australia the first country in the world to give every child diagnosed with cancer access to precision medicine.

Today, more than 2,500 children have been enrolled on the Program and ZERO is leading the world, providing a shining example of how much can be achieved for children with cancer through research-led clinical care.

 **>2500**
children
enrolled

140+
of the
brightest
minds



11 child cancer
centres

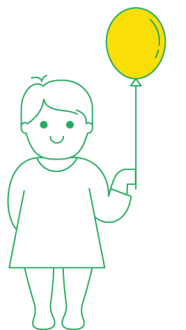


29 research
partners



70%
of children
identified new
potential treatment

2 year survival
Double
vs standard
therapy



But ZERO's impact extends well beyond the lives of children enrolled on the program. It is truly leading the way for how precision medicine works worldwide, with information being shared on protocols and systems for similar programs around the world.

The wealth of data being generated by ZERO is being shared globally, creating an invaluable resource for cancer researchers worldwide, driving many different research programs and international clinical trials. The more that is learned through ZERO, the more its impact grows, accelerating the rate of progress in childhood cancer research for the benefit of all.

ZERO's future

Thanks to Commonwealth Government funding announced in March 2025, ZERO will continue to be available to all Australians aged 0-18 diagnosed with cancer — approximately 1000 patients each year. Additionally, it will be expanded to become available to 19–25-year-olds with newly diagnosed or relapsed paediatric-type cancers — about 300 extra cases per year.

But the future we envisage for ZERO involves more than simply continuing to offer the core

program. To optimise ZERO so it remains world leading and able to deliver the best possible results for children with cancer, its platform needs to be continually enhanced through the incorporation of new technologies and capabilities as they become available.

There are a number of exciting new initiatives in the pipeline which we believe will add significantly to ZERO and will prove to be game changers for the treatment of childhood cancer.

In terms of equity, there is a range of social, economic, legal, ethical, and indigenous research that needs to be done to ensure that ZERO is available equally for all Australian children and young people. Finally, critically, major steps need to be taken to see ZERO embedded into the Australian health system as a permanent, clinically accredited platform.

Precision medicine is the way of the future. It promises to not only improve survival rates, but also improve overall health outcomes for those undergoing treatment and a higher quality of life for survivors. ZERO is changing the whole way childhood cancer is viewed, with implications for children in any country, now and in the future.



Jack's story

Eight-year-old Jack was living a busy and active life in Sydney's Inner West. With mum, Tanya, and dad, Andrew, both working full-time, family life was hectic but not without its fun.

One morning he woke up vomiting and his parents figured he'd caught something. But three days later, it was still going on so they decided to take Jack to hospital to get checked out.

He was given blood and urine tests, then sent home when the results came up clear. But several weeks went past, and Jack was still vomiting.

Tanya took Jack to see a paediatric gastroenterologist who booked him in for a gastroscopy and an MRI, just in case. As soon as the MRI was done, the doctor called.

"He told us he had found a lesion on Jack's brain and to go straight to go straight to the children's hospital," says Tanya.

Jack was admitted to Sydney Children's Hospital, Randwick straight away and put on the neurology ward.

Within two days, Jack was having brain surgery. And while the operation went well, his post-surgery recovery proved challenging.

"You're walking into the intensive care unit, with the sickest kids, and I think that was when the severity of it hit me," says Tanya.

When Jack opened his eyes the first time, they were looking in different directions. He also began swearing, having never sworn before.

"Without that research, we're not going to get anywhere. We just need to get more funding to make sure it doesn't ever stop, because kids like Jack are getting diagnosed every day."

- Tanya, Jack's mum

Tanya, who describes her son as quiet, sensitive and 'an old soul', says it was a lot to deal with.

"I don't know how we got through that week. It was the week from hell."

Tanya and Andrew were told Jack had a brain cancer called medulloblastoma, and would need radiation and chemotherapy.

He was immediately enrolled on the Zero Childhood Cancer Program (ZERO) to have the genetic make up of his cancer analysed. When the results came through, they learnt the cancer was a very aggressive subtype which was growing rapidly. Jack's clinician, Professor David Ziegler, says the results changed Jack's course of treatment.

"When we looked at it under the microscope, when we looked at the scans, it looked like it was just a standard brain tumour that we knew well how to treat. But when we did that genetic analysis, we found that if we gave him the standard treatment, there was a good chance it wouldn't work. So before we even started treatment, we were able to adjust it, giving more intensive treatment."

Six weeks of daily radiation was followed by chemotherapy. Side effects included hair loss and lethargy, and all the while, the daily vomiting continued.

"He had three types of chemo at the highest dose possible. It was quite confronting to hear words such as 'obliterated' and 'blasted' used to describe the harsh protocol Jack was on," says Tanya.

"The worst moment was around cycle two or three. He just burst into tears and said, I don't want to go back; I want to stay with my family. It really wore him down."

Because of side effects such as vomiting and diarrhoea, together with fevers and infections, Jack spent far more time than anticipated in hospital.

One of the hardest things for Jack's parents was trying to calm him down when he had one of his 'episodes'. In a state of half sleep, he would jump out of bed screaming and filled with rage.

Eventually, Jack's treatment ended and today he is tumour free. However, he has been left with a multitude of treatment-related side effects. These include hearing loss, double vision, extreme fatigue, balance and coordination issues, and extensive muscle aches.

"The scariest part is that it has a 40% chance of coming back. I just think it's too high, but there's nothing that we can do except hope and pray," says Tanya.

But she says the progress that has been made with childhood cancer research is 'amazing' and gives them hope for Jack's future.

"Without that research, we're not going to get anywhere. We just need to get more funding to make sure it doesn't ever stop, because kids like Jack are getting diagnosed every day."



"When we did that genetic analysis, we found that if we gave him the standard treatment, there was a good chance it wouldn't work. So before we even started treatment, we were able to adjust it, giving more intensive treatment."

- Professor David Ziegler
Paediatric Oncologist, Kids Cancer Centre
Sydney Children's Hospital, Randwick
Group Leader, Brain Tumours
Children's Cancer Institute





**Minderoo
Children's
Comprehensive
Cancer Centre**

A new era of hope for children with cancer

For 40 years, Children's Cancer Institute has been conducting research into the causes, prevention and cure of childhood cancer. When we opened our lab doors in 1984, survival rates for childhood cancer were not much more than 50%. Today, more than 8 out of 10 children survive. But there is still so much to do to achieve our vision of curing every child with cancer.

In 2025, the Institute will take a significant step towards this vision with the move to the Minderoo Children's Comprehensive Cancer Centre (MCCCC), Australia's first dedicated comprehensive cancer centre for children. Representing a new era of collaboration between clinicians and scientists, the MCCCC promises to deliver tomorrow's care today – transforming experiences and outcomes for children with cancer and their families.

The first centre of its kind in Australia, the MCCCC builds on a 40-year partnership between Children's Cancer Institute, Sydney Children's Hospitals Network including the Kids Cancer Centre, and UNSW Sydney. It aims to deliver the best outcomes for children with cancer through the full integration of research and clinical care.

Featuring world-class facilities and state-of-the-art technology, the MCCCC will include:

- a 900-strong community of dedicated child cancer professionals: clinicians, scientists, and allied health workers
- state-of-the-art, technologically advanced wet and dry laboratory spaces
- education, training and research to develop the next generation of interdisciplinary leaders and workforce

- new inpatient units designed with a child and family focus
- a new outpatient treatment centre with the capacity to deliver a range of therapies, now and into the future.

The Centre will transform the cancer journey for children and their families — from diagnosis and treatment through to survivorship — while at the same time enhancing the capabilities of programs like Zero Childhood Cancer, accelerating research discovery and its translation into improved clinical practice.

Research will span the entire spectrum of childhood cancer, looking at prevention and treatment, along with the impact of cancer on families. Patients and their families will ultimately benefit from improved outcomes, spend less time in hospital, and have access to new clinical trials and a range of cutting-edge treatments.

The Centre will provide opportunities for greater education, leveraging opportunities with UNSW to attract, develop and retain the next generation interdisciplinary workforce.

The influence of the MCCCC will be felt nationally and globally with greater collaboration and partnerships. Children and young people with cancer worldwide able to benefit from the expertise, innovations and research being shared, ensuring that together we can achieve the vision of ending the burden of childhood cancer.



“The Minderoo Children's Comprehensive Cancer Centre will be the first in Australia entirely focused on improving the outcomes for children with cancer by combining globally leading research with exceptional clinical care. This seamless integration will drive research excellence and ensure discoveries swiftly translate from the lab bench to the bedside, locally, nationally and internationally.”

- Professor Michelle Haber AM



A joint initiative of:





**We will cure every child of cancer.
It's not if, it's when.**

Donate today at ccia.org.au/donate

PO Box 351, Randwick NSW 2031
1800 685 686 | info@ccia.org.au | ccia.org.au

