

Annual Review Highlights 2023



Our message to you



Like every one of you, here at Children's Cancer Institute, we believe that a life should be long. That every child should have the chance to grow up, grow old, chase their dreams, and fulfil their potential.

But cancer cuts life short for hundreds of children every year. Our research is focused on finding better, safer treatments for children with cancer so every one of them has the chance to live a long and healthy life.

With many experiencing significant financial pressures, 2023 was not an easy year. Yet, despite its challenges, your belief in our work has allowed us to make extraordinary progress this year.

Our quest for new treatments for children with cancer that are not only more effective but also less toxic led to important developments this year. Among these was a world-first discovery that many more children with cancer than previously realised

could be candidates for immunotherapy, and a breakthrough in the use of nanotechnology to deliver treatment drugs directly to cancer cells.

Research by our Brain Tumours Group led to the launch of a new clinical trial for children with a deadly brain cancer called Diffuse Intrinsic Pontine Glioma (DIPG). We were also thrilled to see two major initiatives launched — the Australian Centre for Targeted Therapeutics, which will focus on developing new treatments for childhood neuroblastoma among other diseases, and the Australian Cancer Research Foundation-funded ACRF Spatial Immune-oncology Research Program, which aims to develop new immune-based therapies for children with cancer.

We saw an important development in the Institute's history with the launch of a new research structure and appointment of two new Deputy Directors. These changes are designed to provide our scientists

with the best possible working environment and will drive great science that maximises our research impact.

2023 was a particularly significant year for the Zero Childhood Cancer Program (ZERO), which we co-lead with the Kids Cancer Centre at Sydney Children's Hospital, Randwick. An important milestone was reached when the 1000th child was enrolled on ZERO in July. Then in December, ZERO was expanded to include children with every type of cancer — regardless of disease type or risk — rather than only those with high-risk cancer, making Australia the first country in the world to offer precision medicine to every single child diagnosed with cancer.

Lastly, we saw main works get underway for the Minderoo Children's Comprehensive Cancer Centre, our new home from the end of 2025. Our long-held dream of working under one roof in a fully integrated way with our clinical colleagues at the Kids Cancer

Centre is coming to fruition, and we couldn't be more excited!

We hope you agree that the impact of our work is significant and far-reaching, with implications for children throughout the world and into the future. Thank you so much for joining us on this journey and for believing that a life should be long.

Together we can and will cure all children of cancer. It's not if, it's when.

Professor Michelle Haber AM
Executive Director, Children's Cancer Institute

Front cover: Lucy was diagnosed with Ewing sarcoma at just three years old.

Financial Report

Statement of profit or loss or other comprehensive income For the year ended 31 December 2023

	2023	2022	2023 (%)
Revenue			
Research	59,158,176	40,897,144	45%
Fundraising	21,376,057	22,770,769	-6%
Interest revenue	563,426	263,527	114%
Other	1,835,205	1,526,098	20%
Total	82,932,864	65,457,538	27%
Expenses			
Research and scientific activities	69,918,375	55,491,158	26%
Fundraising	9,172,172	7,932,966	16%
Support and administration	9,530,309	5,559,695	71%
Other	-	2,407,643	-100%
Total	88,620,856	71,391,462	24%
Surplus/(Deficit) for the year	(5,687,992)	(5,933,924)	-4%

Statement of financial position As at 31 December 2023

	2023	2022
Assets		
Cash, receivables, other financial assets	68,451,118	66,721,462
Property, plant and equipment, intangible assets	9,655,932	9,620,008
Lease asset	3,658,595	6,585,467
Total assets	81,765,645	82,926,937
Liabilities		
Current trade & other payables	6,840,294	6,426,814
Provisions	4,794,661	4,290,951
Other liabilities	37,843,470	33,037,807
Lease liability	1,249,131	2,180,191
Total liabilities	50,727,556	45,935,763
Net assets	31,038,089	36,991,174
Equity		
Reserves	8,842,995	11,514,979
Retained surplus	22,195,094	25,476,195
Total equity	31,038,089	36,991,174

*Total equity includes project-specific research funding received in advance to specifically support identified multi-year research programs.

Our year in review

February

New clinical trial for fatal brain cancer

A new clinical trial for children with a fatal brain cancer, Diffuse Intrinsic Pontine Glioma (DIPG), was launched. Based on ground-breaking research by our Brain Tumours Group, the trial uses each child's own immune system to specifically target DIPG, as well as diffuse midline glioma tumours, through a type of immunotherapy known as CAR-T cell therapy. The trial is named Levi's CATCH in memory of Levi Wheeler, who died from DIPG when he was eight years old.



Minderoo Children's Comprehensive Cancer Centre takes shape

In February, main works got underway for the Minderoo Children's Comprehensive Cancer Centre, which will become the Institute's new home in late 2025. The Centre will deliver integrated, specialist research-led cancer treatment, and educate future research and clinical leaders for children with cancer.

March

Thousands take on 86k for a Cure

In March, our second ever 86k for a Cure event saw over 6000 participants across Australia walking, running or doing 86k of anything to raise funds for our research. Just under \$2 million was raised with many supporters and families of children with cancer connecting with our cause for the first time, engaging this community with their passionate belief in our research.

April

Study paves way to more children benefiting from immunotherapy

In a world-first, our researchers found that many more children with cancer than previously realised could be candidates for immunotherapy, a type of therapy which has been used to great effect in the treatment of some adult cancers. Published in the international journal *Genome Medicine*, this research could lead the way to safer therapeutic options for many children in the future.

May

Nanomedicine research takes a targeted approach

In new research published in the leading international journal *Science Translational Medicine*, our scientists used nanotechnology to develop a 'targeted drug delivery system' to deliver the treatment drug doxorubicin directly to leukaemia cells. Through this targeted approach, the aim is to make treatment more effective as well as much safer to use in children.



June

Donate Your Age

We launched the campaign Donate Your Age for our tax appeal. The new brand-led concept asked supporters to show their appreciation for the life they have lived by donating their age. The campaign, which would go on to win the 'Most Innovative Campaign' at the Fundraising Institute Australia awards, also ran across Childhood Cancer Awareness Month and Christmas appeals, with significant success, recruiting 1451 new donors and raising almost \$200,000.



July

New research structure announced and Deputy Directors appointed

The Institute launched its new research structure and appointed two new Deputy Directors: Associate Professors Paul Ekert and Mark Cowley. Key aspects of the new structure include realignment of our research groups into three Research Themes, and the creation of five Enabling Platforms to facilitate access by our scientists to cutting-edge resources and research capabilities.

One thousand children enrolled in ZERO

A milestone in Australia's medical history was reached in July with the enrolment of the 1000th child in the Zero Childhood Cancer Program (ZERO), Australia's national precision medicine program for children with cancer. Jointly led by our Institute and the Kids Cancer Centre at Sydney Children's Hospital, Randwick and involving every children's hospital in Australia, ZERO is not only positively impacting the lives of those children enrolled, but is changing our whole understanding of cancer in children.

August

New study shows value of drug screening

A study by scientists in the Zero Childhood Cancer Program (ZERO), published in the high-impact journal *Cancer Research*, showed that high-throughput drug screening can provide important insights into children's cancers and how best to treat them. Adding drug screening to genomic analysis for children enrolled on ZERO can help us better understand drug responses, which over time will allow us to better predict which patients are likely to benefit from which therapies.

September

ZERO Dash receives 'Best in Class' at Good Design Awards

A new digital platform developed for the Zero Childhood Cancer Program (ZERO), ZeroDash, won the prestigious Best In Class for outstanding design and innovation at the 2023 Good Design Awards. By streamlining ZERO's data matching and interpretation processes, ZeroDash reduces the time taken to generate a precision medicine report for a child by 70 per cent — a vital step in making ZERO accessible to every child diagnosed with cancer.

Diamond Ball sparkles brighter than ever

We were delighted to welcome 750 of our most special supporters to our annual Diamond Ball in Sydney. Against a backdrop of economic uncertainty and the rising costs of living, the event achieved a phenomenal result, raising almost \$1.7 million, to help us 'move the dial' for kids with cancer.



October

New \$15m frontier technology centre announced

The Australian Centre for Targeted Therapeutics — a collaboration between Children's Cancer Institute, Walter and Eliza Hall Institute, and Monash University — was established with \$15 million in support from the Australian Government's Medical Research Future Fund. The Centre will focus on exciting new 'protein degrader' technology aimed at destroying disease-causing proteins, including those found to drive the childhood cancer neuroblastoma.



November

Professor Michelle Haber AM receives CEO Magazine Lifetime Achievement Award

Professor Michelle Haber AM was honoured for her outstanding contribution to childhood cancer research over the last 40 years with the prestigious Lifetime Achievement Award at The CEO Magazine's Executive of the Year Awards for 2023. The magazine's founder, Chris Dutton, paid tribute to Prof Haber, saying "few have achieved more in improving outcomes for young cancer patients."

December

Precision medicine made available to all children with cancer in Australia

A landmark moment in the treatment of childhood cancer was reached when the Zero Childhood Cancer Program, originally only for children being treated for high-risk cancers, expanded to become available to all children diagnosed with cancer in Australia. This is the first time that precision medicine has been made available to every child diagnosed with cancer, not just in Australia, but in any country in the world.



Lucy's Story

In March 2022, Lucy was a happy-go-lucky three-year-old, enjoying family life with mum (Sarah), dad (Pat), little brother (Billy), and big sister (Grace) who had only just started kindergarten.

One night while Sarah was putting Lucy to bed, she noticed a lump on Lucy's ribcage. A self-confessed hypochondriac, she immediately booked Lucy in to see the family's GP the next morning, already fearing the worst.

'Our appointment with the GP was at 8:30AM, and by about 9:30AM, we'd had an urgent chest x-ray done,' says Sarah. 'An hour later, our GP called me and said "Go to Sydney Children's Hospital. Pack a bag and go now."

As soon as Lucy arrived at the Emergency Department, she was seen by an oncologist. Ten days later, after multiple scans and biopsies, the diagnosis was confirmed as Ewing sarcoma, an aggressive type of childhood bone cancer.

Because the cancer had already spread to her lungs, Lucy was initially given a very poor prognosis, leaving Sarah and Pat devastated. She was admitted to the cancer ward and began her first cycle of chemotherapy straight away. During this first cycle, she suffered a mild seizure and febrile neutropenia which resulted in her staying in hospital for 12 days.

When the head oncologist examined Lucy, he was surprised to find that he could no longer see evidence of the tumour on her rib cage. After only

three days of chemotherapy, Lucy's tumour shrunk so significantly that it was no longer obvious.

'He told me that Lucy had responded extremely well to her first cycle of chemo and that this was an incredible result,' Sarah says. 'This gave us some hope that Lucy might be able to beat the odds that were stacked against her.'

Following nine cycles of chemotherapy, Lucy had surgery to remove what was left of her tumour, as well as the three ribs that it had attached to. When the removed tumour was biopsied, Lucy's family received more good news — only eight active cancer cells were found — the tumour was all but dead. Again, this was an incredible and rare result for this aggressive type of cancer.

While the chemotherapy that Lucy was given seemed to be effectively killing her cancer, the side-effects it caused were horrendous. She was constantly neutropenic, which resulted in many long hospitalisations. She suffered severe mucositis (inflammation of the membrane lining the gastrointestinal tract), caught COVID twice, and had multiple bacterial, fungal and blood infections. She also had seizures and suffered life-threatening damage to her heart and lungs.

'She was so sick, and in so much pain. We were in hospital all the time,' says Sarah.

After surgery, Lucy was given five more cycles of chemotherapy, as well as radiation to her lungs. It was then that Lucy suffered another seizure

(caused by neurotoxicity from one of her chemo drugs) — this one far more serious than the first.

Over the next 24 hours, Lucy made a full recovery, but Sarah and Pat were faced with a very difficult decision. They could try the same chemotherapy again, this time with a treatment called methylene blue to try to prevent another seizure, or trial a different chemo combination that may not be as effective at killing Lucy's cancer.

After weeks of research and discussions with their oncology team, the decision was made to continue with the original chemotherapy plan, with the assistance of methylene blue.

'For those six days, I just sat on the end of her bed and stared at her', recalls Sarah, 'If Lucy were to have another seizure there was a possibility that it may cause lasting brain damage, so I was watching very closely for any warning signs that she was having an adverse reaction to the chemo.'

To their enormous relief, the methylene blue treatment worked. Lucy completed her chemo protocol and remained seizure-free. But not long after leaving hospital, Lucy's oxygen levels dropped dangerously low, and they were forced to return. The doctors tried to stabilise Lucy's oxygen levels, initially without success.

'I was terrified. Nothing was working. Lucy was declining rapidly and within 24 hours she was not able to breathe at all without a high-flow oxygen machine,' Sarah says.

Lucy was diagnosed with pulmonary hypertension — a potentially fatal heart and lung condition which can lead to heart failure. She was referred to a cardiologist and immediately began treatment to try to reverse the damage being done to her heart.

Eventually, after five long months of being attached to oxygen 24/7, Lucy once again beat the odds. Incredibly, her pulmonary hypertension had spontaneously resolved and her heart function had returned to normal. She was no longer at risk of heart failure.

'We've come close to losing her so many times, but she somehow keeps surviving everything that's thrown at her,' says Sarah. 'She takes it all in her stride and really just gets on with it. I don't know where she finds her strength.'

When Lucy was first diagnosed, she had been enrolled on the Zero Childhood Cancer Program (ZERO), at which point samples were taken and sent for genomic analysis. Now, the results were back and it was good news: Lucy's cancer cells

had a known genetic alteration that could be targeted with a drug. 'The oncology team told me that we could now do maintenance chemo, because they'd found a gene they could target,' explains Sarah.

While no guarantee could be given that the drug would keep Lucy's cancer at bay indefinitely, Sarah says she found the results from ZERO comforting. 'They've got something they can act upon, and if the cancer does come back, they can put her back on the drug immediately while we discuss what to do next.'

In September 2023, Lucy finally finished maintenance treatment. She is now getting three monthly scans, as well as chest x-rays in between, to check for any signs of cancer. In January, she started kindergarten.

'We're certainly not out of the woods. For the first two years, there's an extremely high risk of relapse. If Lucy can get to that point, that would be a good start, but if she can get to five years, then perhaps we will have more confidence in her long-term survival.'

Sarah is a strong advocate for childhood cancer research, and recognises that her family has reaped the rewards of past research made possible by donations.

'We're benefitting from the research that people have donated to in previous years. If your child gets diagnosed next year, you'll need the research that's happening right now.'





A life should be long.



Donate today at ccia.org.au/donate

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