



### Lucy is full of life thanks to you

Lucy was just 3 years old when she was diagnosed with Ewing sarcoma, a rare and aggressive cancer. She was given just a 30% chance of survival.

Lucy had to endure a gruelling treatment protocol which resulted in many side effects. She lives with the high possibility of relapsing sometime in her future. Fortunately, researchers from the Zero Childhood Cancer Program were able to identify a drug that could target one of Lucy's cancer genes. This medicine was included in Lucy's maintenance protocol, with the hope it would prevent her from relapsing.

"It's hard to believe there was a 70% chance that Lucy wouldn't be alive today. Yet here she is, a vibrant 6-year-old who is full of life. Despite daily health issues and the ever-present threat of relapse, Lucy's resilience and zest for life shine through, inspiring everyone around her. Every moment with her is a blessing and a powerful reminder of the life-saving impact of research."

Sarah, Lucy's mum

### Thank you for your support



On behalf of everyone here at Children's Cancer Institute, and from all the children and families you are helping, I want to thank you for your support throughout 2024.

In 2024 we celebrated 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 is thanks to kind and caring people like you, who continue to ask this question, that we have been able to continue to make great progress towards our vision of curing every child with cancer.

As you'll see in this report, your support is helping us make breakthroughs and change the model of care for children with cancer in ways that previously we could only have dreamt of.

I hope you're proud of your contribution. With your continued support we are closer than ever to a future where every single child will survive their cancer. Where every child will have the opportunity to grow up and grow old. Because, like you, we believe that a life should be long.

Warm regards,

Professor Michelle Haber AM

Executive Director

Children's Cancer Institute

## You're changing the model of care for children with cancer



In June we published world-first results from the Zero Childhood Cancer Program (ZERO) in the top international journal Nature Medicine, showing that precision medicine is superior to standard treatment in children with high-risk cancer. As a result, ZERO is changing the entire model of care for children with cancer in this country.

Since 2017, ZERO, which is led in partnership by Children's Cancer Institute and the Kids Cancer Centre at Sydney Children's Hospital, Randwick, has been providing in-depth genomic analysis for children with cancer across Australia.

The study, which looked at 384 children with high-risk cancer enrolled on ZERO, showed that the precision medicine approach taken by ZERO significantly improves outcomes, both in terms of clinical response and survival. 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.

Considering these were children with highly aggressive cancers which, in many cases, had already failed to respond to standard therapy, these results are extraordinary and show that ZERO is truly life-saving for some children. Put simply, more children are alive today thanks to supporters like you.

"We've already shown that precision medicine can help identify new treatment options for many high-risk patients. Now we've shown that it not only can shrink their tumours, but also lead to a significant improvement in long term survival for those patients."

Professor David Ziegler, Chair of Clinical Trials for the Zero Childhood Cancer Program





#### World-first discovery for high-risk neuroblastoma

"This work opens up entirely unexplored avenues for the development of new therapeutic approaches for neuroblastoma, and quite possibly other cancers as well."

Professor Murray Norris, Co-Leader, Experimental Therapeutics & Molecular Oncology Group Our researchers have made a world-first discovery that a gene known as RUNX1T1 is essential in the development of high-risk neuroblastoma, a cancer that is responsible for the deaths of more Australian children under the age of five than any other cancer.

The research found, 'knocking out' or silencing this gene completely prevented the development of tumours in the lab. Excitingly, it also appears to make these cancer cells more visible to the body's immune system, opening up the potential of immunotherapy as a way of targeting these devastating tumours.

There was also evidence that RUNX1T1 plays an important role in some adult cancers – opening up the possibility of a new approach to treating a number of other cancers in both children and adults.



Grace was just eight months old when she was diagnosed with neuroblastoma. She had a 9cm x 7cm tumour which was not only preventing her from moving her legs but also compressing her right lung.

After eight rounds of chemotherapy and multiple surgeries, doctors were able to remove all active cancer cells. Now 16 months old, mum Brooke says Grace is back to being 'a normal little kid'. But with the ever-present threat of relapse and side effects. Brooke knows our research is vital.

# New hope for children with one of the most aggressive leukaemias



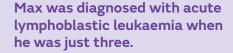
Our research team has found that a new-generation drug developed to treat T-cell acute lymphoblastic leukemia (T-ALL), an aggressive leukaemia that has few treatment options, has 'excellent potency' both as a single agent and in combination with other therapies.

Led by Professor Richard Lock, Head of the Cancer Biology Theme, and Associate Professor Charles de Bock, Team Leader in the Functional Genomics of Leukaemia Group, the research involved developing a drug known as ACHM-025 to specifically target T-ALL cells, then testing this drug in preclinical models to evaluate its safety and efficacy.

"Results of this initial preclinical testing have been extraordinary.
Compared to the 70+ new agents we've tested in our lab over the last 20 years, this one is up there with the best."

Professor Richard Lock, Theme Head, Cancer Biology The results were nothing short of spectacular. In the study, models of some of the most aggressive forms of T-ALL were cured with this single drug administered once a week for three weeks. ACHM-025 was also tested in combination with standard of care chemotherapy, with results indicating that even low doses could lead to an effective treatment.

The study offers fresh hope that a new and much-needed treatment option may be on the horizon for this aggressive disease, that is not only effective but with fewer side effects.



Despite initial treatment, our research detected remaining cancer cells that indicated he was at high risk of relapsing. As a result, doctors were able to adjust his treatment.

Max turned 10 last year and is celebrating five years post treatment. He got to spend time travelling to Greece and Italy with his family and continues to enjoy learning more about Jiu Jitsu and Kickboxing. He just reached the next belt in his grading and got to have private lessons at the birthplace of Muay Thai during travel in Thailand!



Every month extraordinary people like Peter give children a second chance to enjoy a childhood free of cancer



Here in Australia, we have more than 1000 children and adolescents being diagnosed with cancer every single year. And each week, three young lives will be lost to the disease. For those who survive, life is never the same again. Two thirds will suffer long-term health problems caused by the very treatment that saved their life.

Like you, we believe every child deserves to live a healthy and happy life. Your monthly gift will make that possible.

When unforeseen circumstances arise, your monthly gift provides our researchers with the stability and security they need to continue delivering crucial research for children with cancer. Knowing we have consistent monthly support means we can commit to new pilot studies, explore new technologies, and fund long-term research initiatives that bring us closer to finding a cure for every child with cancer.

Peter, a supporter of the Institute since 2005 became a regular donor in 2013.

"I find it heartbreaking that children can get cancer through no fault of their own. I've been fortunate in life, and I want to give back. Knowing my monthly donations provide stability to researchers working on targeted therapies gives me hope that we'll not only find a cure but potentially one day, even prevent children from getting cancer in the first place."

Become a monthly donor today and you'll join a community of compassionate supporters, just like Peter, who are determined to create a better future for children with cancer.



To be part of this incredible mission, scan the QR code to set up your monthly donation, or contact us via

email regulargiving@ccia.org.au phone 1800 685 686 or visit ccia.support/ImpactRG

#### Your incredible impact in 2024



177 researchers
39 students

114 research publications





active clinical trials supported by our research

782
children enrolled on
the Zero Childhood
Cancer Program, now
over 2000 in total.





400 collaborators worldwide

33 awards and honours





49 lab tours 237,000 social media followers





## Together we will cure every child of cancer. It's not if, it's when



Scan the QR code to find out more about the impact of your support

To find out more visit ccia.support/Impact2025 1800 685 686 | info@ccia.org.au | ccia.org.au

