

zero

CHILDHOOD CANCER

Care to Cure

The momentous journey towards curing childhood cancer



The Sydney
children's
Hospitals Network
care, advocacy, research, education

Four words no parent should ever have to hear: “Your child has cancer.”

Currently, for children with the most challenging forms of cancer, there is still very little hope.

Declan (cover) was diagnosed with high-risk stage four neuroblastoma just before his fourth birthday. He is currently still in treatment for his disease at Princess Margaret Hospital, Perth* and has also undergone treatment at Sydney Children's Hospital, Randwick.

*Princess Margaret Hospital, Perth is due to be replaced by Perth Children's Hospital as the State's dedicated children's hospital.

Tragically, in any one year, more than 950 Australian parents will hear those devastating words. **More than 950 children and adolescents in Australia are diagnosed with cancer every year.**

More devastating still, almost **three Australian children or adolescents with cancer die every week**. Without medical research, so many more young lives would be lost.

Prior to the 1960s, childhood cancer was almost always fatal.

Today, due to enormous advances in medical research, the overall survival rate has risen to 80%. However, some types of aggressive childhood cancers still have much lower survival rates.

But even an 80% survival rate is nowhere near good enough... and cancer remains the most common cause of child death from disease in Australia.

It is the three children a week currently dying from cancer that we are determined to save.

With the launch of the **Zero Childhood Cancer** program, Children's Cancer Institute and the Kids Cancer Centre at Sydney Children's Hospital, Randwick will move significantly closer to this goal.

Critically, **Zero Childhood Cancer** will benefit all children with cancer, because for those children who do survive, 70% will suffer lifelong side effects because of toxic treatments: the best we currently have to offer. Thirty per cent of these children's side-effects result in chronic conditions including heart conditions, cognitive disorders, metabolic disorders, infertility or secondary cancers not caused by the primary cancer but by the very treatment given to cure.

The learnings from **Zero Childhood Cancer** will help to find safer, more effective treatments for all children with cancer so they can survive healthier and for longer.

zerochildhoodcancer.org.au

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At just five months old, Brydee (above and page 16) was diagnosed with leukaemia. Now almost two, she is still in treatment at Royal Children's Hospital, Melbourne.

A world first

The **Zero Childhood Cancer** program is a globally-leading initiative: a personalised medicine program that aims to improve childhood cancer survival rates. The goal is to achieve 100% survival for every child with cancer in the foreseeable future.



Led by Children's Cancer Institute in partnership with the Kids Cancer Centre at Sydney Children's Hospital, Randwick (part of The Sydney Children's Hospitals Network), **Zero Childhood Cancer** is the most exciting childhood cancer research initiative ever undertaken in Australia. It aims to tackle the most serious cases of infant, childhood and adolescent cancer, and it gives hope to children with the highest risk of treatment failure or relapse.

The program will involve the detailed laboratory analysis of each child's unique cancer cells, to help identify the drugs most likely to kill their specific cancer. Scientists and doctors will then work collaboratively to identify and deliver the most effective treatment plan, specifically tailored to suit each child's individual disease.

On average more than 200 children and adolescents in Australia will be diagnosed each year with a cancer type that has less than 30% survival, either on first diagnosis or after relapsing from their disease. It is these children – including those suffering from the most aggressive forms of childhood brain tumours, sarcomas, infant leukaemias and neuroblastomas – who will benefit from the **Zero Childhood Cancer** program.

The challenge in curing every child is that each child's cancer is unique, which means they respond differently to anti-cancer treatment. These treatments are harsh on developing bodies: hospital wards are full of children suffering as much from the side effects of treatment as they are suffering from cancer.

Even among the 80% of children who will survive their cancer, almost half will suffer in adulthood from the side effects of toxic chemotherapy drugs currently available (most cancer chemotherapies used today have been in use for more than 40 years).

The challenging commercial reality is that the pharmaceutical industry does not focus on childhood cancer, choosing instead to focus on the larger commercial markets of adult cancers. For this reason, pharmaceutical companies do no dedicated research into childhood cancers and have never developed a drug specifically for childhood cancer.

Personalised cancer therapy, tailored to each child's cancer, with its own genetic characteristics and individual response to anti-cancer drugs, holds the exciting promise of one day pushing childhood cancer survival rates to 100%.

Byron was diagnosed with cancer at 17 months old. Despite relapsing after his initial treatment at Royal Children's Hospital, Brisbane*; Byron is now seven and is doing well.

*The Royal Children's Hospital, Brisbane and Mater Children's Hospital, Brisbane were replaced in 2014 by the Lady Cilento Children's Hospital.

From pilot study to national rollout

Zero Childhood Cancer will offer the opportunity of a personalised treatment plan to all children with cancer in Australia who have less than 30% chance of survival.

The first stage of the **Zero Childhood Cancer** program commenced in 2016, with a pilot study for high-risk young cancer patients in New South Wales. Originally this pilot study was planned for 12 young patients, however more than 30 have been enrolled in the program as of December 2016 due to the high demand by clinicians and parents. During 2016, the pilot study has enabled development and testing of the platform required to establish the personalised medicine pipeline involving:

- establishment of a central reception process for cancer samples to the Tumour Bank
- determination of optimal tumour sample size, shipping conditions and pilot site logistics to inform rollout of a national trial
- molecular profiling including whole genome sequencing, in partnership with the Garvan Institute of Medical Research
- drug screening with a customised anti-cancer drug library
- drug efficacy testing methods using biological models.

Following successful completion of the pilot study, a national clinical trial involving at least 250 children will open in 2017, however, it is likely that up to 400 children will be enrolled by the completion of the trial to benefit from the personalised research and clinical program.

When fully implemented, **Zero Childhood Cancer** will be offered to all children throughout Australia who are at highest risk of relapse or treatment failure.

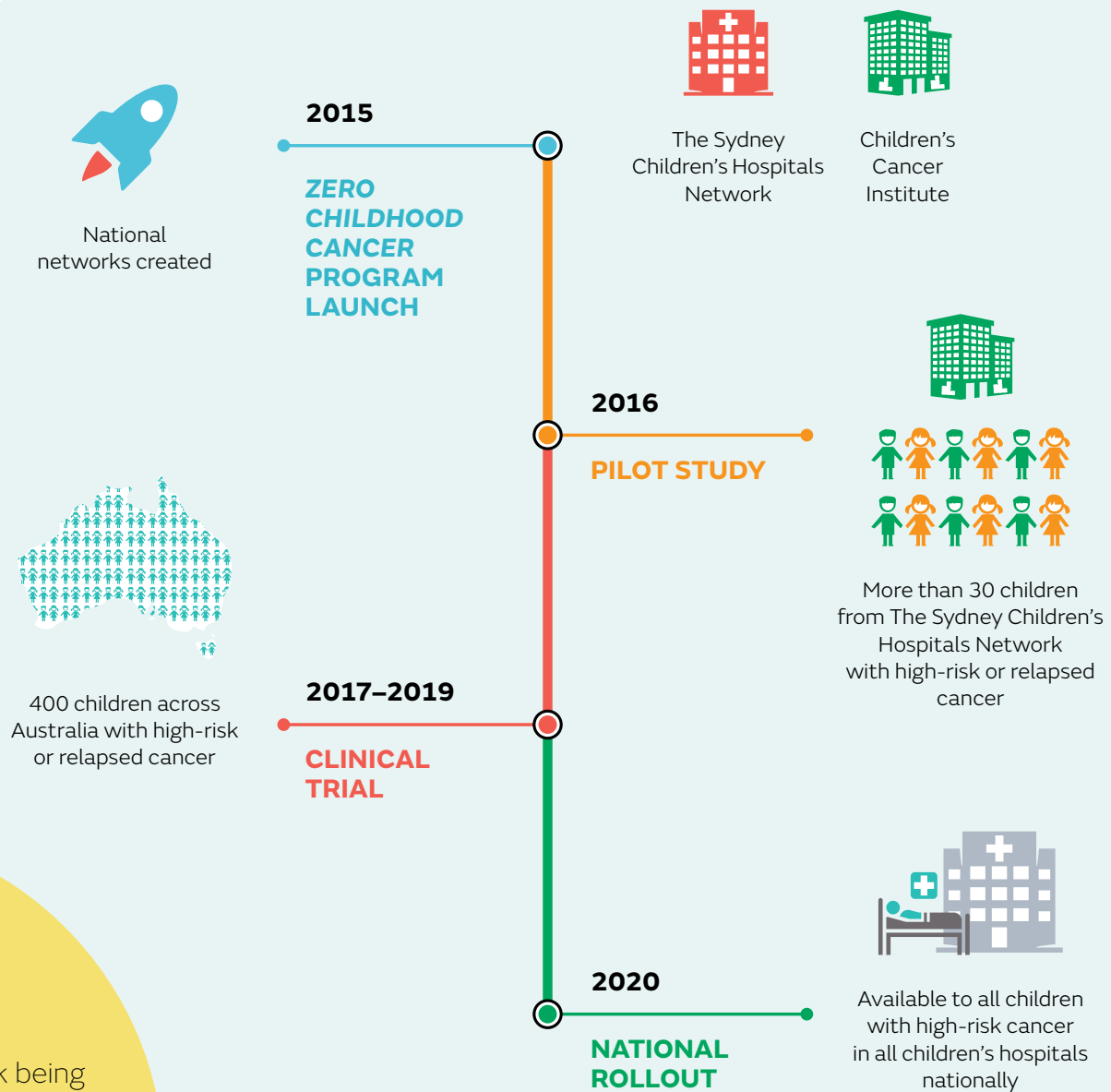
Once it is rolled out across the country, the program will enable all newly-diagnosed, high-risk childhood cancer patients in Australia, and all children who relapse following treatment, to have their therapy tailored to the specific genetic and biological characteristics of their individual tumour. Through the data generated, the national trial will build an evidence base to demonstrate that a personalised approach to treatment has the potential to improve outcomes for individual children, reduce the burden to the healthcare system, in turn driving advances in the gold standards of healthcare delivery.

The Zero Childhood Cancer program will revolutionise the way treatment decisions about childhood cancer are made.

Of the 80% of children who now survive their cancer, more than 70% will suffer in adulthood from the side effects of toxic chemotherapy drugs; 30% will have serious chronic side-effects.



The journey of the program



“There is no greater work being done in medicine than what’s being done here. What could be more important than aiming to have zero childhood cancers?”

Malcolm Turnbull MP,
Prime Minister of Australia,
speaking about the
Zero Childhood Cancer
program





Meet Isla

Age 6, just completed treatment for
T-cell acute lymphoblastic leukaemia.
Sydney Children's Hospital, Randwick.

A transformational philanthropic campaign

Zero Childhood Cancer is an innovative and translational program that offers the very real opportunity to research and implement a revolutionary new approach for childhood cancer treatment. But it is complex and expensive.

To enable the national rollout of **Zero Childhood Cancer**, we need at least \$58 million of funding to ensure the required equipment, facilities and people are in place. These funds must be raised on top of current research requirements.

To date, Children's Cancer Institute has successfully secured commitments of \$42 million from government bodies and funding partners. The scientists and researchers embarking on this visionary program are now turning to the community for support.

This fundraising campaign is a joint initiative of Children's Cancer Institute and Sydney Children's Hospital Foundation. It is a national, landmark campaign that has harnessed unprecedented collaboration between two of the nation's leading children's charities.

Supporters of the **Zero Childhood Cancer** program will have the opportunity to be recognised publicly.

But far greater than any gift recognition will be the knowledge that each person and company who supports this program will share in the victory of accelerating childhood cancer survival rates closer to 100%.

The campaign to support this transformational program aims to raise \$12 million, by engaging the generosity of community-spirited corporates, and compassionate philanthropists Australia-wide.



A collaborative powerhouse

Children's Cancer Institute and the Kids Cancer Centre at Sydney Children's Hospital, Randwick are jointly recognised as international leaders in the field of childhood cancer research and treatment.

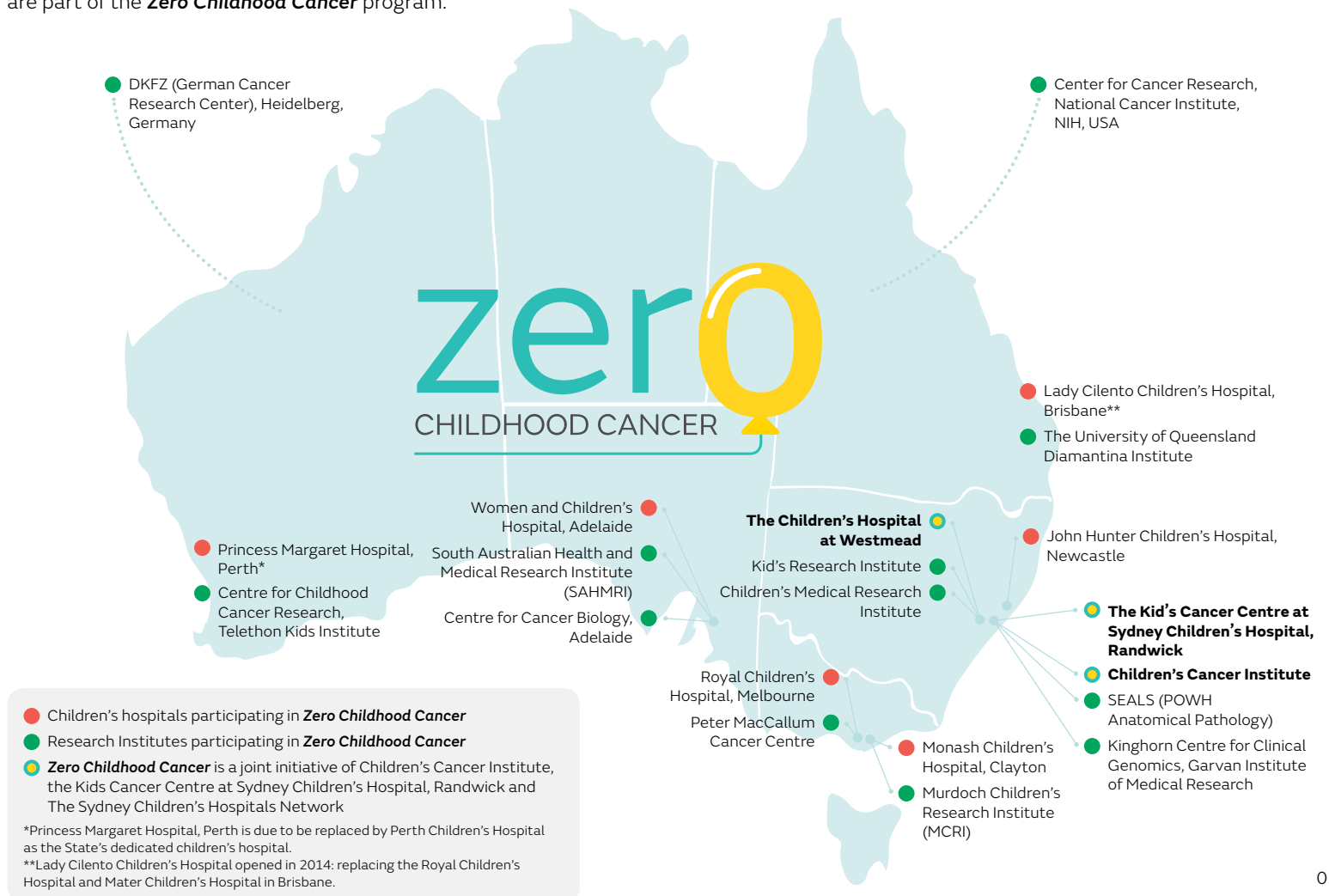
The **Zero Childhood Cancer** program will involve collaboration with major research centres in Australia, USA and Europe. The program will utilise the best-of-the-best expertise in Australia's paediatric hospitals and research centres and will draw on a host of stellar medical and research establishments throughout the nation.

The core research facility for the **Zero Childhood Cancer** program will be located at Children's Cancer Institute. Childhood cancer research specialists from institutes across Australia will contribute to and share results at every stage of the program.

The clinical trial program will be led by the Kids Cancer Centre at Sydney Children's Hospital, Randwick in conjunction with clinical collaborators in every major city in Australia. Decisions on the precise personalised treatment advised for each child will be agreed by a panel of research and clinical experts representing the best capability and knowledge in Australia, USA and Europe.

ZERO CHILDHOOD CANCER COLLABORATION MAP

The **Zero Childhood Cancer** program will involve all paediatric oncology units across Australia who treat the 950 children and adolescents who are diagnosed with cancer every year. These hospitals will work collaboratively with a range of key Medical Research Institutes both nationally and internationally who are part of the **Zero Childhood Cancer** program.





Meet Nixon

Age 2, recently completed treatment
for a rhabdoid tumour.
The Children's Hospital at Westmead.

Nixon's journey

When Nixon's mum Brodie was 32 weeks pregnant, doctors noticed a large lump on her baby's head during a routine ultrasound.



No diagnosis could be made without a biopsy, so the mum-to-be was transferred to The Children's Hospital at Westmead immediately for close monitoring, and just over a week later, little Nixon was born.

Brodie and her husband Nick were devastated when tests on their newborn showed he had a malignant tumour the size of an adult fist growing on the side of his head.

Nixon was just one week old when he underwent a biopsy. The surgery was risky, as he could bleed out due to the invasive procedure his tiny body would endure.

While the five-hour surgery was a success, it would be an agonising week-long wait before mum and dad received the results. When the biopsy results came back, they confirmed the family's worst fear: Nixon was diagnosed with cancer. It was an extremely rare type of tumour known as a rhabdoid tumour. A few days later, at just two and a half weeks old, Nixon started chemotherapy.

In the coming months, little Nixon would endure 30 rounds of chemo, six surgeries, 12 blood transfusions and 11 platelet transfusions. **"That time was such a blur for our family,"** Brodie recalls.

"We had a newborn who had cancer, and a toddler to contend with all while we spent more than 20 weeks living at The Children's Hospital at Westmead."

After a couple of rounds of chemotherapy, to the relief of his family, his tumour started to shrink. Following four rounds of chemotherapy and at four months of age, Nixon had surgery to remove what was left of the tumour. He completed his final six rounds of chemotherapy by January 2016 when he was declared NED (no evidence of disease). Since his operation to remove the tumour, Nixon has undergone numerous procedures to graft skin and heal his wound. These surgeries are ongoing.

Nixon recently had a follow up scan, and doctors are happy with the results so far.

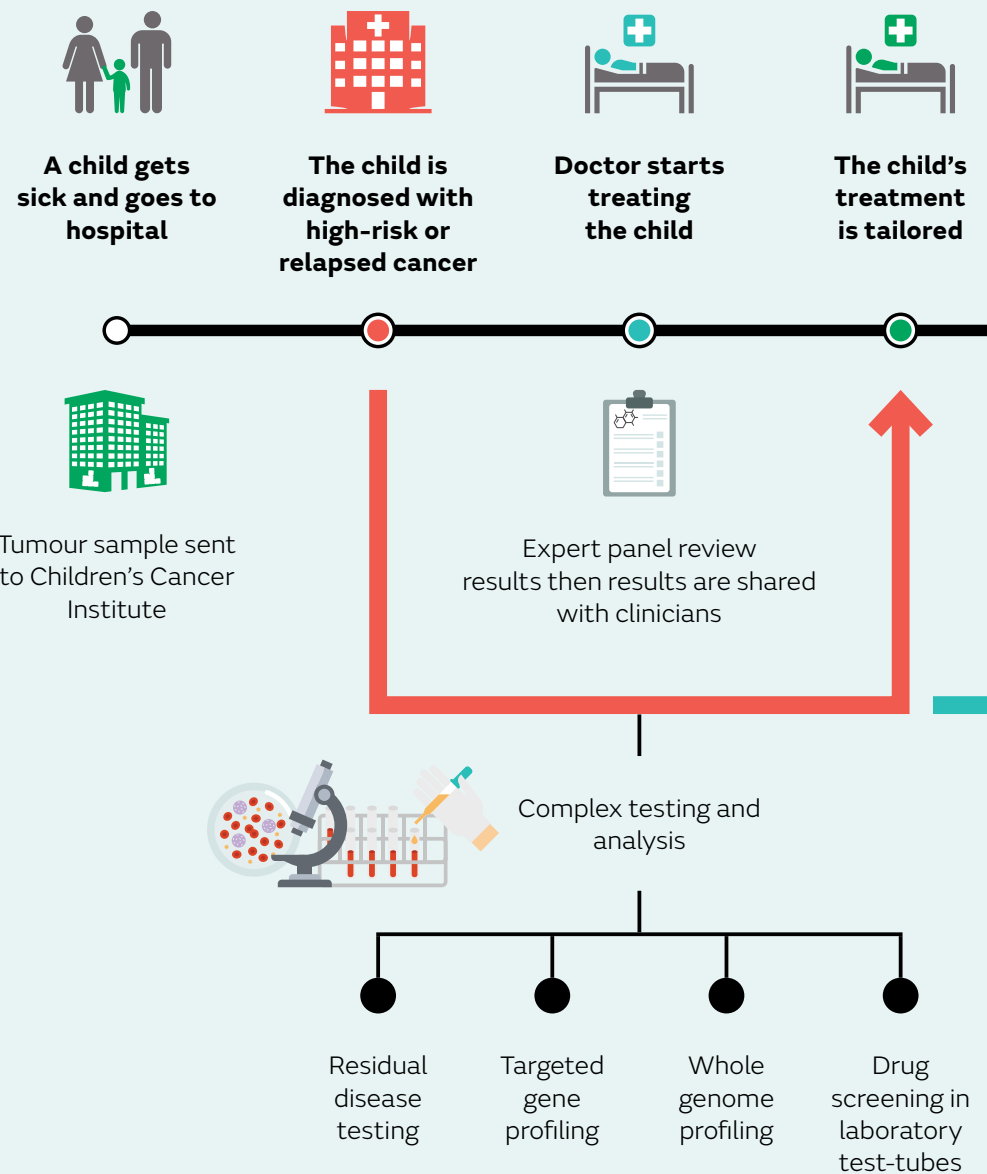
Nixon's family are immensely grateful that past research ensured there were viable treatment options for their son. As Brodie sees it: **"Nixon has come this far due to research. Research is the key to unlocking a cure and improved treatment options for children's cancer. And we all need and dream of a cure!"**



Nixon, undergoing cancer treatment at The Children's Hospital at Westmead.

The journey of a child

Each child enrolled in the **Zero Childhood Cancer** program will have their tumour samples sent to Children's Cancer Institute where a series of complex tests will be completed to enable the development of a tailored treatment plan. The goal is to deliver better treatment, fewer side effects and a greater chance of survival.





**The child's
treatment
is refined**



**Better outcomes
with fewer side
effects**



More complex
testing



Results sent
back to the
hospital



Drug screening
in biological models



Isla's journey

Isla's mum Sarah had taken her four year old daughter to the doctor because she had been snoring and experiencing pain in her knees for some weeks.



Sarah and her husband Darrin had put it down to enlarged adenoids and growing pains. Other than her snoring and knee pain, Isla was a happy, healthy child. She was in day care, attending school orientation and had been to swimming lessons the day before the shocking news came.

Darrin recalls that moment two years ago as if it were yesterday. The GP had asked to see him and his wife as soon as possible. "When we saw our GP," he says, "we knew it wasn't good news and I remember what she said like it was five minutes ago: **'Isla has leukaemia, you have to go to Randwick tonight, they are expecting you.'**"

They rushed their desperately ill child to Sydney Children's Hospital, Randwick that night and Isla's treatment began the next day.

Little Isla had been diagnosed with T-cell acute lymphoblastic leukaemia: a particularly aggressive form of leukaemia. The next few weeks, then months, would be a blur of tests and treatments. Isla's physical appearance changed dramatically from the steroids. Her family's life was turned upside down. Sarah and Darrin, both police officers, returned to work after about a month working

opposite shifts so one could be with Isla 24 hours a day and the other with their other four daughters as much as possible.

Although Isla is still having treatment, Sarah and Darrin recall the first nine months as especially scary, intense and traumatic: emotionally and physically for Isla in particular, and the whole family.

Isla particularly feared the general anaesthetics and having her chemotherapy port accessed to allow the chemotherapy to be administered.

"We are realistic, but optimistic," says Darrin.

*"We know that we will live in fear of every bruise, of every ache and pain dreading the leukaemia has come back, but we have hope that she will be cured. And we have that hope because of the study and research of Children's Cancer Institute. We have been amazed by the determination, compassion and passion by everyone involved in the **Zero Childhood Cancer** program to achieve a 100% cure rate for children's cancer."*



Isla (above) during cancer treatment at Sydney Children's Hospital, Randwick.

Isla (above left) with two of her four sisters, Lily and Ainsley.

Budget and funding partners

Already, the **Zero Childhood Cancer** program has harnessed widespread support and attracted significant funding from the Australian and New South Wales governments, esteemed academic research agencies, foundations and visionary philanthropists.

The fundraising campaign is a joint initiative of Children's Cancer Institute and Sydney Children's Hospital Foundation. It is a national, landmark campaign that has harnessed unprecedented collaborative fundraising energy.

ZERO CHILDHOOD CANCER PROGRAM COSTS AND EXPENDITURES 2015–2020

NATIONAL PROGRAM TOTAL COST	\$58.1M
Operating expenditure	\$33.0M
Capital expenditure	\$25.1M
CONFIRMED FUNDING TOTAL	\$41.7M
Confirmed operating funding	\$16.6M
Confirmed capital funding	\$25.1M
UNFUNDED TOTAL	\$16.4M
Projected competitive grants and renewals from funding partners	\$4.4M
Zero Childhood Cancer Capacity Campaign	\$12.0M

Life-saving, personalised treatment for kids with cancer: delivered through a collective of 19 hospitals and research centres Australia-wide.



Journey with us to **cure** childhood cancer

Ultimately, **Zero Childhood Cancer** will move us closer to **one day, curing every child with cancer.**

We need **YOUR** help to find the cure for childhood cancer.

Please give a gift to help find a cure for childhood cancer.

Zero Childhood Cancer is the most innovative and exciting medical research program ever launched in Australia. It has real potential to rapidly change the course of childhood cancer research and treatment in Australia – and worldwide.

“This is a moment in time. We’re on the edge of an opportunity we’ve been building towards for many years. We’re ready to expand this program so every child with aggressive cancer in the country can benefit. For that, we need your support.”

“Time is precious. We aimed to have 12 children on this pilot study but we already have 33. These children have the most aggressive cancers and sadly, even since the study commenced, some have passed away. For them, we haven’t got to a cure soon enough.”

“We urgently need the resources to open the national clinical trial next year.”

Professor Michelle Haber AM
Executive Director
and Head of Program,
Experimental Therapeutics,
Children’s Cancer Institute



“From our many years of research, we know that each child’s cancer is unique and that a one size fits all treatment approach doesn’t work.”

“Most of the treatments used today are non-specific poisons, meaning they hurt the normal cells almost as badly as they hurt the cancer cells. Our wards are full of children suffering as much from the side effects of treatment as they are suffering from cancer.”

*“The **Zero Childhood Cancer** program will arm us with better knowledge of which drugs target the cancer cells, and not the normal cells. We believe this will give children with cancer the best chance of survival and the best quality of life.”*

Professor Glenn Marshall AM
Senior Oncologist,
Kids Cancer Centre,
Sydney Children’s Hospital,
Randwick
Head of Translational
Research and Molecular
Carcinogenesis Program,
Children’s Cancer Institute





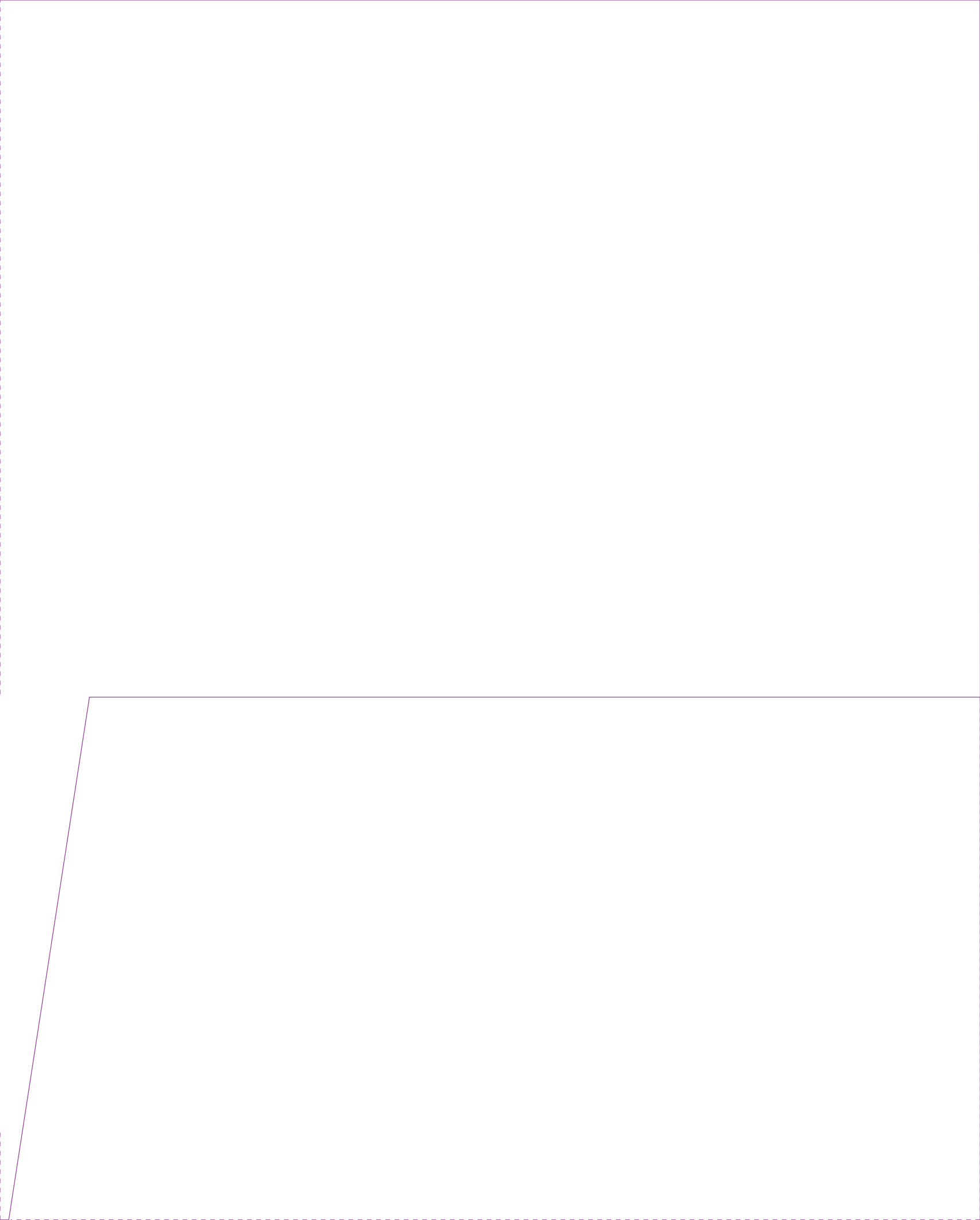
Tyler (above) was diagnosed with rhabdomyosarcoma at the Royal Children's Hospital, Brisbane* on his second birthday. 2016 marked Tyler being cancer-free for five years.

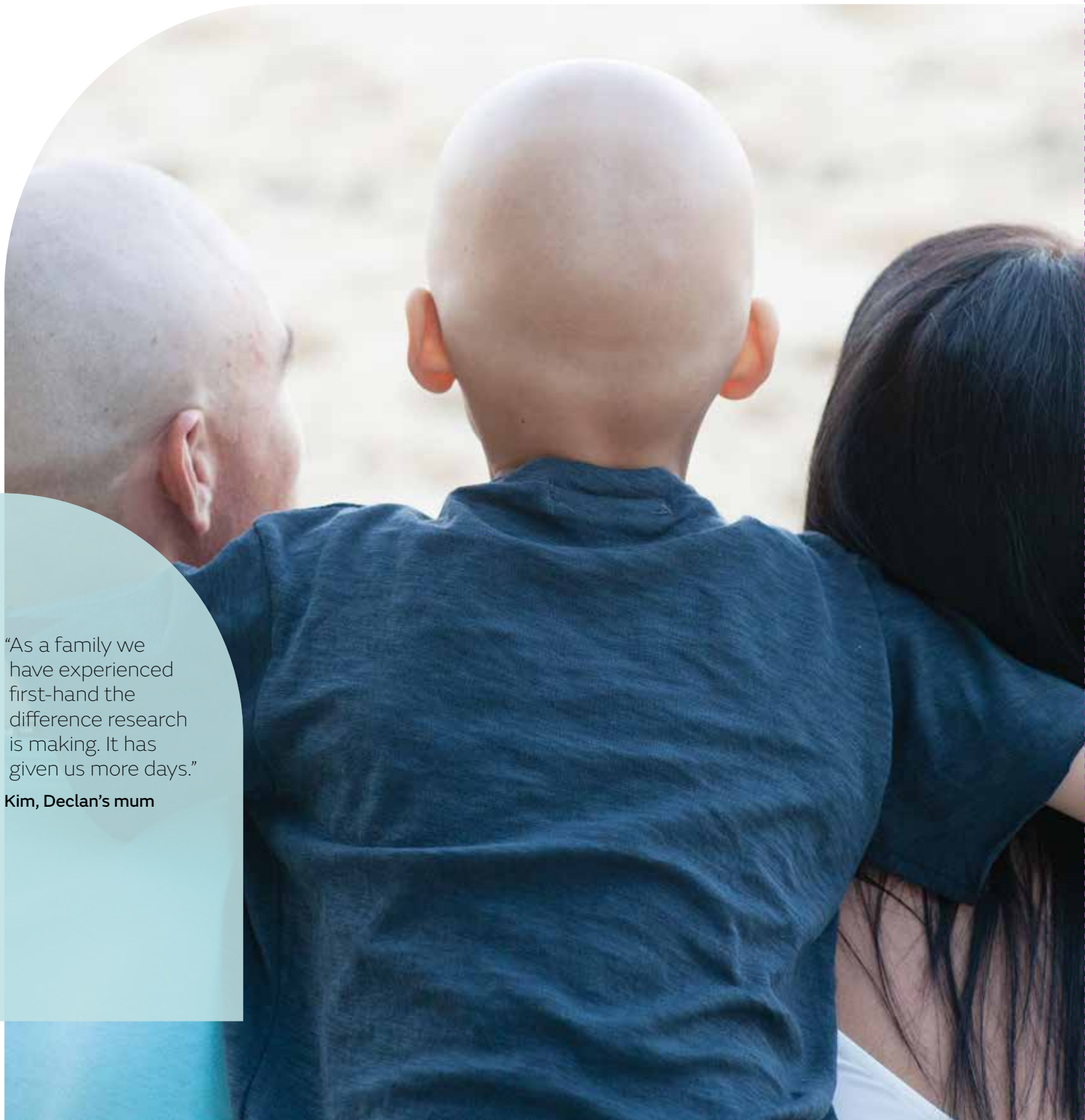
*The Royal Children's Hospital, Brisbane and Mater Children's Hospital, Brisbane were replaced in 2014 by the Lady Cilento Children's Hospital.



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“As a family we have experienced first-hand the difference research is making. It has given us more days.”

Kim, Declan’s mum

