Thank you for helping us beat childhood diseases.

2016 THE YEAR IN REVIEW.
## Contents

3.......... Foreword  
4.......... ProCan™: leading the fight against cancer  
5.......... What ProCan means for children like Fred  
6.......... Bringing brilliant minds together to beat genetic disease  
7.......... Why do we need CATG?  
8.......... Eye Genetics update  
9.......... Thanks for telling us what matters to you  
10.......... Conditions like epilepsy disrupt lives and futures  
11.......... How your donations help  
12-13 ...... Community Fundraising  
14.......... Discovery Day

Cover images: Willow, Max, Elizabeth and Indi  
Right: Amy and Sophie
Foreword

From Professor Roger Reddel, Director of Children’s Medical Research Institute

As we enter 2017, I take a moment to reflect on some amazing opportunities in the year ahead. CMRI is leading the way globally in key areas, including the fight against cancer and treating genetic disease. That’s because of people like you. Your support gives us access to the latest technology and the ability to employ brilliant minds in the fight against serious childhood diseases. The pace of our scientific research is continually accelerating, and we are working equally hard to increase the speed with which those discoveries reach the clinic and benefit children and their families.

Thanks to your support, one day we will defeat childhood diseases.

Here’s what we’ve achieved together in 2016...

Roger Reddel
Lorimer Dods Professor and Director
Children’s Medical Research Institute
In September we launched an international program to change how we fight cancer. Currently cancer kills 8 million adults and children globally every year.

Because of your support, ProCan will save lives by making cancer diagnosis faster and more accurate. This will empower doctors with the information needed to choose the best treatment option for each individual patient, with fewer side-effects. We will analyse 70,000 cancers over the next 5 years.

In future, when a patient is diagnosed with cancer, their cancer can be analysed within 36 hours and compared to the vast store of information that will exist. By giving doctors a diagnosis of the exact type and subtype of cancer, a personalised treatment plan can be recommended giving the patient the greatest likelihood of success. We expect ProCan will improve survival rates and reduce suffering from almost all types of cancer.

This, by any measure, is revolutionary.
What ProCan means for children like Fred.

Many childhood cancers are difficult to diagnose, and coming up with the best treatment requires educated guesses, as Fred Bellette’s story shows.

“Fred was only 3 days old when I noticed a lump when I was changing his nappy – but no one knew what the problem was,” said mum, Kate.

“Ten days later, we were flown from Alice Springs to Adelaide for a biopsy and MRI. People tiptoed around the ‘C’ word, calling it a ‘mass’ or a ‘lesion’. In the end, they pronounced it a rare but non-malignant mass and sent us home. I felt something was wrong when it began to change.”

The long journey to diagnosis included tests in Adelaide, then in Sydney (Westmead). At age 6 months, Fred’s cancer was finally diagnosed and treatment with surgery and then chemotherapy began, continuing, with terrible side-effects until he was 2 years old.

“It’s the most un-empowered situation for a parent – a mixture of despair and disbelief, it’s just so horrific.”

Once its 5-year project is complete, ProCan will speed up diagnosis and treatment planning for children like Fred. This means fewer tests, fewer disruptive trips to capital cities for diagnosis and treatment and more importantly, faster action to make sure the cancer is treated early, before more intensive treatment is required.

“The day after Fred’s first birthday was the day he had to start a new, more rigorous, more risky, chemo regimen. I had seen this protocol take the smile from children’s faces for months while they were on it. I was grief stricken. This was (also) the day we met the team here at Children’s Medical Research Institute, and that was the moment we found out about the research efforts that would give hope to families like ours.”

Kate said, “This is the place that gives parents like me hope for Fred’s future, and a hope that no other child’s story has to echo ours.”

You’ve helped give hope to families like Fred’s. Thank you.

HERE’S WHAT YOU’VE HELPED US ACHIEVE IN 2016:

A purpose-built facility to house ProCan’s sensitive instruments was opened at CMRI in Westmead in September, with Dr David Gillespie representing the Prime Minister, Malcolm Turnbull at this historic event.

ProCan was the first international signatory to the US Cancer Moonshot program. We are proud to play our part in this global collaboration in leading the way of this research that will not only improve diagnosis and treatment using existing medicines, it will accelerate the search for more cancer cures globally.

We have been recruiting leading experts to work in the ProCan lab. Associate Professor Rosemary Balleine has joined this team recently as Group Leader, Cancer Pathology Group to help us achieve our aim of replacing most current cancer tests with a single, faster and more accurate ProCan-based test.
Bringing brilliant minds together to beat genetic disease:

The Centre for Applied and Translational Genomics (CATG)

There are over 6,000 genetic diseases, each rare, but they devastate families and are a significant burden on the health care system.

1 in 20 children suffers from a birth defect or genetic disease. Many families have no answers, because international resources are too scarce to research each disease individually. Realising that together, we can improve understanding and treatment of genetic diseases, we established CATG and became a Paediatrio partner to share resources and knowledge. Paediatrio is a key collaboration between all children’s research institutes and hospitals in Sydney to coordinate efforts and improve outcomes for children.

Through Paediatrio, which launched at Parliament House in December, we are combining our expertise with that of clinicians in Sydney’s leading hospitals to methodically investigate rare diseases and bring treatments to patients sooner. DNA sequencing helps us screen for genetic errors, but much additional research is needed to determine how it all works and to develop treatments. CMRI and our partner institutions will research different genetic diseases in a coordinated way, based on our areas of expertise.

2016 ACHIEVEMENTS:

We helped launch Paediatrio in December to help bring treatments and cures to children sooner.

Our world-leading embryology researchers, led by Professor Patrick Tam, completed decades of research that will help predict how genetic errors cause birth defects and other genetic diseases.

Professor Robyn Jamieson was appointed Professor of Genetic Medicine and thanks to your support of her work, she can help children suffering from genetic eye disease.

We opened a new genetic research facility called Vector and Genome Engineering Facility (VGEF) to help improve the understanding of rare genetic diseases and develop gene therapy cures for all types of genetic disease.
Families affected by genetic disease first ask ‘Why?’ and then ‘What can we do about it?’ The CATG will help answer both those questions.

Justine loved being a grandmother, so when she heard her daughter Charlotte was expecting again, she was over the moon.

“Charlotte was a natural mother, and we were all excited,” she said.

Alexis was born happy and healthy, and was home within 24 hours. Justine planned her trip down to Melbourne where Charlotte and her husband Ian lived. In a few days she’d be there, cuddling her new granddaughter.

Then the phone call came. “Mum, Lexy’s gone.”

Everything went fuzzy and Justine rushed to pack and reach Melbourne as fast as she could. Then Justine organised the funeral and took care of everything she could. And she wanted to understand how this had happened. She talked to doctors around the country, and had CMRI send DNA to Denmark for testing.

“I wouldn’t have thought about genetic disorders if it hadn’t impacted on our family,” said Justine.

“If research had not been done, we wouldn’t have known about the disease that killed Lexy. We would never have narrowed it down. Genetic research gave us an answer so we could know ‘why’ and stop blaming ourselves.”

Justine knows that research is the only way to stop more children like Lexy dying. Apart from accidents, the leading cause of death in children under 4 is birth defects and genetic disease. And for ages 4 to 14, it’s cancer.

Children’s Medical Research Institute is working hard to address these diseases. CMRI’s researchers focus on four main areas of research, and have already achieved world-class excellence in cancer, neurobiology, birth defects and gene therapy. Their progress shows that no disease, whether it is cancer, or a genetic disorder, is unstoppable.

Justine couldn’t be there to shower her newly born granddaughter with kisses and gifts, but by leaving a gift in her Will to CMRI, she can leave a legacy of love for all children.

Why do we need CATG?

If you are intending to make a Will and would like more information on how to go about it you can contact us at bequests@cmri.org.au
Eye Genetics update

The Eye Genetics team has learned why a genetic error in one gene is causing blindness in the White family. Now, using this knowledge, they are working to develop gene therapy approaches that could not only help the White family, where three children face blindness, but also pave the way for gene therapy of other eye diseases.

More than one family’s sight is at stake. Retinal diseases are the leading cause of blindness and affect 1 in 5,000 people. Right now, all three children in the White family are slowly losing their vision due to a genetic disease that affects the retina. However, the gene therapy tools we are developing have the potential to correct many other genetic eye diseases and help yet more families.
Thanks for telling us what matters to you.

We are extremely grateful to those who completed last year’s survey, as it helps us keep you up to date on what’s important to you.

YOUR TOP THREE MOTIVATIONS FOR SUPPORTING MEDICAL RESEARCH:

1. I think that medical research is the only way new treatments and cures can be developed.
2. I want to accelerate the speed with which medical research can develop new ways for doctors to help those who are sick.
3. It provides me with a way to help people.

THE MOST POPULAR RESPONSES FOR WHY YOU SUPPORT CMRI:

22% It’s the only children’s medical research institute in Australia that focuses on genetic research

19% I know that CMRI is founded with and relies on community support from people like me

33% The majority of my donation goes directly towards children’s medical research

37% CMRI’s track record proves my donation is used to create the building blocks for future medical treatments

THE AREAS OF RESEARCH YOU ARE MOST INTERESTED IN:

- Cancer: 31%
- Neuroscience: 28%
- Gene Therapy: 26%
- Embryology: 15%
Riley was a normal boy until age 6, when he began to experience moments where he stopped and stared at nothing. Riley was diagnosed with Absence Epilepsy and prescribed a combination of drugs. Nothing worked. The intensity of seizures grew. At one point, he had four seizures in one day and was hospitalized. An EEG showed 46 seizures in 4 hours, and that was just a normal day. Social life was affected; school was affected – the whole family was affected.

“If I could help others not go through this, I’d do it in a millisecond,” mum, Ruth says. Riley and his family visited Professor Phil Robinson’s laboratory at CMRI to learn about the new treatments for epilepsy under development — treatments that may one day help those who do not respond to current medications, kids like Riley.

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“You’re helping to make sure kids like Riley will one day have the treatments they need.

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“I’m glad to see this group of people focused on epilepsy,” Riley’s dad, Damien, said. “I’m glad someone is trying to find something better and not forgetting about us.”

There may not be a treatment for Riley or kids like him right now but we are working to make sure those treatments exist in future.

WHAT YOU’VE HELPED ACHIEVE IN 2016:

Our new medications for epilepsy have been proven to be safe in concept, and are effective in pre-clinical tests.

We’ve taken a major step towards making these new medicines potentially useable in humans, by making them water soluble.

Conditions like epilepsy disrupt lives and futures

Epilepsy, along with other neurological and neurodegenerative disorders limit potential and cause life-long suffering to children, adults and their families. For the last 20 years, our research has been taking a wholly new approach to understanding how the brain works, what controls the signals between brain cells, and how to correct those signals when something goes wrong.

Thanks to you, we can continue world-leading work to understand endocytosis, a key signalling process used by brain and other nerve cells. This research has led to the development of new medicines that, once refined, could help the 1 in 3 people with epilepsy who do not respond to current medications to control their seizures.
In November, we sent a Christmas decoration to our supporters and asked them to send a message of support for Professor Robinson and his team. We received hundreds of responses...

Thank you

Thanks for all your good wishes and the time you have personally taken to write the messages and send them to us. Those messages inspired not only me, but all the other researchers at CMRI. And they made a delightful Christmas tree in the lab!

We are all grateful for what you’re doing to keep this important work going. We look forward to the next stages of getting our new epilepsy therapies ready for clinical trials.

Professor Phil Robinson,
Head of Cell Signalling Unit

How your donation helps

Research Spend by Disease 2016

![GameChangers pledge regular financial support by making a monthly donation. This allows us to plan ahead. If you would like to find out more please contact us on 1800 436 437 or by email at info@cmri.org.au](image)

- **Neuroscience 18%**
- **Genetics 19%**
- **Embryology 13%**
- **50% Cancer**

$40 will help find the cause of deadly cancers. You can pay to analyse the DNA code of a gene that could be involved in multiple cancers.

$60 helps us to uncover the causes of rare diseases. Help buy vital laboratory supplies to understand the causes of rare diseases.

$80 can identify DNA gone wrong. Your donation will help buy the special materials needed for extracting cancer DNA to help us find out what turns an innocent cell into a killer.
Jeans for Genes Day 2016 was a great success! CMRI staff along with CMRI committee members and countless volunteers set up stalls across the country, helping to raise money and awareness of our work. Jeans for Genes Day continues to be one of the most popular fundraising events of the year for CMRI and for the Australian public. Supporters signed up in their thousands to help raise much needed funds by selling merchandise or holding events in their workplaces, at their clubs and among their friends. The total funds raised this year have topped more than $1.8 million.

Excellence Ball

This star-studded event was held at the Four Seasons Sheraton hotel in Darling Harbour and was attended by over 300 guests. Among those present was 2015 Jeans for Genes ambassador, Jodi Gordon, with her mother, Bronwyn Gordon; Sydney Swans player, Josh Kennedy, and his wife, Ana; actress, Emma Lung, and her husband, Henry Zalapa. Former Olympic swimmer, Daniel Kowalski, was the master of ceremonies.

Sally Obermeder, a cancer survivor, was the 2016 Jeans for Genes ambassador. “I’m a mother. I have been through a cancer battle. I remember when I was sick, I was thinking ‘my goodness, thank God this isn’t my child’ and then I remember thinking ‘thank God this isn’t any child’, and that for me is the most important thing – when you talk about children and sickness, it should just never be in the same sentence,” Sally said.

Paralympian, Ellie Cole, shared her experience of childhood cancer and the amputation of her right leg, with the packed room. “The hardest thing I found with my cancer journey and being sick as a child was how my family were affected. My siblings were almost shifted to the side while I was going through my treatment...it was really tough. To know that CMRI is doing work to put an end to that is really positive,” Ellie said.
Mudgee 50th Anniversary

It’s not every day a rural fundraising committee reaches its 50th birthday. CMRI’s Mudgee Committee achieved just that on Sunday, 13th November. The occasion was celebrated in the gardens of Mudgee’s historic Heaton Lodge.

During its 50 years, the dedicated committee has raised close to $500,000 which has enabled scientists at CMRI to research childhood diseases.

“It is amazing to be part of something that has achieved so much in terms of research and development,” Committee President, Kate Baker, said. “I am always amazed at the Mudgee community’s very generous support.”

Fundraising in the community
Discovery Day

Ever wondered what a research laboratory looks like?

You are invited to see CMRI’s work first hand by visiting the Institute, and hearing more about our research and watching scientists at work.

If you would like to attend a Discovery Day contact us on 1800 436 437 or email info@cmri.org.au to make a booking.

Other ways to get involved:

If you want to do more to help defeat childhood diseases, here are some ways to show your support:

• Become a GameChanger and be part of the team that keeps CMRI moving forward. Your regular monthly donation is an essential ingredient in creating a brighter future for children in Australia.
• Consider leaving a gift in your Will.
• Jeans for Genes® Day: On the first Friday in August, wear jeans and make a donation, sell merchandise or organise a fundraising event.
• Challenge events: Run, ride, swim or take on a team challenge. Run in the City-To-Surf or other events to raise money for CMRI’s vital work.
• Great Cycle Challenge™: Get a team together, set a riding target and fundraising goal and use our online platform to track your progress. Ask colleagues and friends to sponsor your team as you ride throughout October.
• Join us at our annual Jeans for Genes Excellence Ball, or come along to another function. Visit cmri.org.au/events
• Purchase Christmas cards and gifts from our online shop at shop.cmri.org.au
• Volunteer: Help behind the scenes at events or talk to us about other volunteering opportunities.

Visit our website cmri.org.au or contact us on 1800 436 437 to learn more.
1. Minister for Medical Research, Pru Goward at the opening of the Vector & Genome Engineering Facility
2. CMRI Research Excellence Winners: Dr Tony Cesare, Professor Robyn Jamieson & Associate Professor Tracy Bryan
3. Cell Bank lab
4. Mr Len Ainsworth & dignitaries at the opening of the Ainsworth Tower
5. ProCan team
6. 2016 Masquerade Ball hosted by the Penn family
7. ProCan lab in action
8. Scientists at work in the ATAC Facility
9. Scientists in the Genome Integrity Unit

If you would like to support us today please click here