

Under the microscope



NEWS FROM INSIDE THE INSTITUTE FOR OUR SUPPORTERS

SPRING EDITION 2021

Unflagging Support for Jeans for Genes

Once again, even in the most difficult of times, Australians have shown their unwavering support for CMRI's research by donating more than \$1.5 million to this year's Jeans for Genes campaign.

Many areas of Australia were locked down due to the pandemic on Jeans for Genes Day, Friday 6th August, but that didn't dull the community's enthusiasm.

We saw businesses hosting Bake it Blue morning teas over Zoom, TV presenters dancing in denim, police showing their support, kids selling their artwork, and whole regional communities turning their towns blue— what an impressive effort!

It was especially heartening for our scientists who can't all be in their labs right now. CMRI's Director, Professor Roger Reddel AO, said, "It is so encouraging for our researchers to see Australians everywhere supporting us, especially during such tough times, and the faith that people have in our work."

"Thank you for improving the health of future generations of Australians, and for helping children living with serious genetic diseases who urgently need treatments to be developed. Along with our researchers, the parents of children living with these conditions are also immensely grateful for your support and the hope it brings for a better future."



Finding cures for children's genetic diseases



From the Director

Welcome to this Spring Edition of our Under the Microscope newsletter.

Throughout the pandemic, CMRI has made every effort to create working arrangements that ensure the safety of our staff, research students and the wider community, while continuing critically important work. During the current lockdown, extended restrictions in our Local Government Area since late July have required us to severely limit our laboratory work. We have, however, found ways to continue much of our research away from the laboratories, with many of our teams using this opportunity to spend time analysing large amounts of data that had already been collected, writing papers and planning new experiments.

In recognition of the significant discoveries that can result from analysing "big data", CMRI's ProCan team has been named a finalist for the Research Australia Data Innovation Award. Congratulations also to A/Prof Tony Cesare for his ARC Future Fellowship award. This prestigious fellowship recognises Australian research excellence at the highest level, and will help support his team's investigations into how our cells make faithfully accurate copies of their DNA.

As always, thank you to our generous community for continuing to get behind Jeans for Genes. We are immensely grateful. Likewise, our Great Cycle Challenge has started well ahead of October, raising over \$900,000 so far. To participate, please sign up at greatcyclechallenge.org.au

If there is a silver lining to the months of lockdowns and restrictions endured by many Australians, for us it is the increased recognition in the community of the value of medical research as the key to health. Thank you for your loyal support of our work enabling children to have a healthy childhood, and lead long and healthy lives.

Sincerely,

Roger Reddel AO
Lorimer Dods Professor and Director, CMRI



Send some Christmas cheer this year. Buy your cards, gifts and more from our online shop.

—Visit shop.cmri.org.au



CMRI's Associate Professor Tony Cesare has received a prestigious Australian Research Council Future Fellowship to discover key processes in DNA replication. He aims to better understand the fundamental biological processes that protect human genomes.



Research Update

25 years of work rewarded



— Professor Ian Alexander (left) and Dr Grant Logan (right) - their grant idea was recognised as top ranked in Australia

CMRI's gene therapy project which aims to save infants' lives has been named the top-ranked National Health and Medical Research Council (NHMRC) Ideas Grant for 2020, in what the lead researcher describes as "proof that we weren't just dreamers and symbolic of the power of the genomic revolution".

Professor Ian Alexander, and lead investigator Dr Grant Logan, were awarded the 2020 NHMRC Marshall and Warren Ideas Grant Award on 16 June.

Prof Alexander is Head of the Gene Therapy Research Unit, which he formed more than 25 years ago.

"The gene therapy field is coming of age and earning the scientific respect that it has increasingly deserved."

Gene therapies are 'genetic medicines' where healthy copies of genes are delivered into diseased cells to replace or repair faulty genes and therefore treat or even cure disease. The delivery vehicle for the healthy gene is called a vector (typically AAV).

Westmead has become a globally leading centre in using AAV-based vectors in gene therapy.

"This award is linked to the fact that we are at the front end of seeing the impact of the genomic revolution," Professor Alexander said.

"It heralds our ability to treat disease by gene transfer - the most stunning example being the treatment of the life-threatening disease, Spinal Muscular Atrophy (SMA) in infants.

"We are now trying to go beyond that. The success of the SMA trials is not the end, it's just the beginning. There is so much powerful science that can be leveraged by this progress."

The team is now looking at ways to engineer more efficient AAV vectors to better treat SMA and potentially other genetic diseases.

— For more details visit: <https://agtc.org.au>



There are only a few weeks left until the annual Great Cycle Challenge begins! Hop on your bike and raise money for vital cancer research being done at CMRI. Join the thousands of people who have registered to start the month-long event in October.

—Learn more here: <https://greatcyclechallenge.com.au>



Research Australia has recognised CMRI's ground-breaking ProCan cancer project. ProCan has developed a world-first data analysis workflow to enable reliable comparison of results from cancer samples that were analysed by different instruments over a long period of time.



Dr Pablo Galaviz Vilchis is a Data Scientist in CMRI's Bioinformatics Team. His role is to take data generated in the labs and transform it into meaningful information to answer complex biological problems.



Q & A

Dr Pablo Galaviz Vilchis
Data Scientist,
Bioinformatics Team

What is your background?

I studied Physics in Mexico and went to the University of Jena in Germany for my PhD. My research topic was numerical relativity and the simulation of black hole collisions. After that, I came to Australia to work on cosmological models at Monash University and the collision of stars at Macquarie University. I've also worked in gaming and automation engineering.

What attracted you to science?

I was curious about how things

work, experimenting with electronics since one of my uncles had a TV repair shop. When I was eight, I started studying my older brother's physics textbook. I liked it so much that I decided to be either a physicist or an F1 driver. I did not have the resources to become a racing driver; so I focused on science, and now I follow racing on TV.

What is your favourite part of your job?

My favourite is starting a new project and learning about something I don't know. I like to think about ways to solve a problem. At CMRI, I have extra motivation. I know that what we do here will eventually have a great impact on the health of children and young people.



Committee & Community update

Regional Communities Save the Day

While much of Australia was in lockdown on Jeans for Genes Day – some of CMRI's regional fundraising committees were able to save the day and hold extraordinary events.

One of the largest fundraising drives was held by the Mudgee Committee who have surpassed \$107,000 in a town of 12,000 residents.

Canberra Committee held an event at The Hellenic Club and raised more than \$35,000 at the formal dinner.

The Quirindi Committee set up a Jeans for Genes stall in a local store in Gunnedah and sold cupcakes.

Gerrigong Committee held a successful trivia night with 100 people at the Gerroa Fisherman's Club.

The small but influential Goulburn Committee convinced 18 schools across the district to do a Jeans for Genes event and, combined, they raised more than \$9,000!



— Jenny Johnston, Gilgandra committee

Gilgandra Rallies Once More

Once again, the phenomenal Jenny Johnston and the entire Gilgandra community have blown our minds and their own records by raising \$11,000!

Every year Jenny visits every single shop in the small regional town as well as local schools, dropping off coin boxes and selling merchandise. Battling cancer hasn't stopped her, and we're so grateful for her dedication to our cause. Thanks to every one of the 4000 people in Gilgandra!



— Mudgee committee (left), Canberra committee (right)

Fight with Five Partners Virtual Launch



— Fight with Five DNA strand.

Being in lockdown didn't stop the enthusiastic Western Sydney business community from coming together virtually to support the launch of CMRI's Fight with Five Partners.

Fight with Five is an initiative which sees businesses partner with CMRI by donating \$5,000 to the important work being done in our labs. In return, they have their logo featured as part of the DNA strand on our website and are offered tours of the labs to meet scientists and learn how their contribution will change the lives of future generations.

The Fight with Five Partners launch saw everyone come together online for the Virtual DNA Strand reveal, and to share their motivation behind why they believe research is vital.

CMRI's Director, Prof Roger Reddel AO, thanked the partners for supporting research at what is a difficult time for many individuals and businesses.

CMRI Board Member James Wakim, who invited many within his network to join the Fight with Five campaign, thanked those who accepted, stating, "I feel blessed to be a part of it."



— Thank you to our Fight with Five supporters.

Other business participants spoke of the "privilege" of supporting research, about the importance of giving back and how "blown away" they were by the work being done at CMRI. Many also reflected on how incredible it was that CMRI was in the heart of Western Sydney, in Westmead, and that this was an opportunity to help future generations.

Thank you to Artline, Parramatta Chamber of Commerce and Loxley on Bellbird Hill for supporting the Fight with Five initiative with in-kind donations for the partnership inclusions.

To find out more or become a partner go to www.cmrijeansforgenes.org.au/fightwithfive



Please donate by phone or fax:
P.+1800 436 437 F.+61 2 8865 2801



Please donate online at:
www.cmri.org.au/donate



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— Harper's family on Jeans for Genes Day.

Burpees for research

Harper was diagnosed with HK1 and CCNA1, a neurological genetic condition that could lead to severe developmental and speech delays, intellectual impairment, vision problems and seizures. Harper's family organised a fun 1km travelling burpees challenge to raise awareness and funds for childhood genetic diseases. They were overwhelmed with generous community support, raising over \$9,600 for research.



— Redlands school on Jeans for Genes Day.

Kids supporting Jeans for Genes

A huge congratulations to Redlands School for raising \$8,984 for Jeans for Genes. Despite the COVID-19 lockdown, they did an amazing job of engaging their entire school community online by posting videos and posters in the school newsletter and online learning platform with links to their fundraising page on social media.

Thank you for your generosity. Your valuable support allows us to continue our work on the most serious problems affecting the health of our children. Every dollar counts.

Will you be a supporter to help find cures for children's genetic diseases?

Yes, please accept a single gift of
\$ _____
or
 Yes, please accept my monthly gift of
\$ _____ per month

Please find enclosed:

Cheque Money Order
(payable to Children's Medical Research Institute)

Please debit this card:

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Please send me more information about including a gift in my Will to Children's Medical Research Institute.

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Thank you for your generous support.

— We hope you enjoy reading the newsletter and are always happy to receive your ideas and feedback. Please let us know at research@cmri.org.au