

# under the microscope

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## Today's scientists for tomorrow's children

The Children's Medical Research Institute (CMRI) is an independent organisation committed to unlocking the mysteries of disease. Our scientists investigate conditions such as birth defects, cancer, and epilepsy. Our philosophy is that major advances in prevention and treatment come from research into the fundamental processes of life. Our work is made possible by our community of supporters and Jeans for Genes®.

50 YEARS OF  
**DISCOVERY**  
CHILDREN'S MEDICAL RESEARCH INSTITUTE

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*Brodee (centre) aged four years old with older sisters Courtney and Jamie McKenna*



## Newman's Own funding

The Children's Medical Research Institute (CMRI) has been awarded almost \$50,000 by the Paul Newman Foundation for research into Rett syndrome.

Long-term CMRI supporters, the McKennas, know very well the importance of this funding—their six year old daughter Brodee has Rett syndrome.

"Brodee was officially diagnosed with Rett syndrome at two years and two months of age" said Mrs McKenna, "when she was 13 months old she would play with toys or hold a biscuit, but everything she picked up went straight into her mouth. We were trying to stop her from doing this then she stopped holding toys altogether. She hadn't started crawling or walking and we noticed she was grinding her teeth."

This is a typical story for parents of girls with Rett syndrome. Their daughters may never walk, are likely to suffer epileptic seizures, and are at risk of scoliosis (curvature of the spine). The average life expectancy of a person with Rett syndrome is less than 50 years, during which constant care and attention are required in every aspect of daily living.

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# Director's desk



Research grant awards are highly competitive. The decisions about what research should be supported are usually made at arms length from government, through national organisations such as the National Health and Medical Research Council, and the Australian Research Council. For researchers to be successful in the fiercely competitive processes of these organisations, the judging panel must be convinced that the researchers' ideas will have a very worthwhile outcome, and that the researchers already have a record of high-level achievement.

It is therefore particularly pleasing that this edition of *Under the Microscope* has articles about a number of Children's Medical Research Institute researchers who have recently been awarded funding. In addition to the much-needed funds, this is also a recognition of excellence from the nation's top judging panels.

It is often said that the work of our researchers—building the foundations for major improvements in the health of our children and our children's children—is priceless. But it is completely dependent on funding, and the competitive funding accounts for a third of what is needed. The global economic crisis will undoubtedly present major challenges for us in the year ahead, but with your support we can make sure that this transforming research continues at full pace.



Professor Roger Reddel



Prof Phil Robinson, Dr Mark Graham, Dr Scott Cohen, Associate Prof Tracy Bryan, Prof Roger Reddel and Prof Patrick Tam have received over \$3 million in competitive national grants

## \$3 million in funding

With recent economic turmoil tightening purse-strings it's a relief to know that the Australian government will continue to support vital medical research.

The Children's Medical Research Institute (CMRI) has been awarded over \$3 million in funding from the National Health and Medical Research Council (NHMRC), and the Australian Research Council (ARC) for research over the next three years.

Prof Phil Robinson and Dr Mark Graham from CMRI, and their collaborators around Australia, received almost \$2 million from the NHMRC. The money will fund two research projects that are closely linked to the development of new drugs to treat epilepsy, and a third project to better understand the cellular mechanisms for repairing damaged DNA.

"These grants will allow us to really push ahead with research that will help us develop new treatments for epilepsy and cancer. We're really pleased to get the go ahead" said Dr Graham.

Associate Prof Tracy Bryan and Dr Scott Cohen were awarded over \$600,000 by the NHMRC for research to determine the structure of telomerase, an essential step towards developing drugs that target telomerase to treat cancer.

"I am very encouraged that the NHMRC has the foresight to fund 'high risk-high reward' research projects like ours. We are now in a position to pursue this exciting goal" said Associate Prof Bryan.

Prof Patrick Tam of CMRI and his collaborator, Prof John Christodoulou from The Children's Hospital at Westmead, were jointly awarded almost \$500,000 in NHMRC funding to research the disease processes in Rett syndrome and related disorders.

CMRI was also eligible to apply for funding through the ARC for the first time this year because of its closer links with the University of Sydney.

Dr Graham and Prof Robinson received \$300,000 from the ARC to determine the role of two types of natural protein modifications in brain function. "Results from this research will be of great benefit in designing compounds to fight diseases of neurotransmission such as epilepsy, depression and schizophrenia" said Dr Graham.

Prof Tam received \$412,000 from the ARC for research into how the essential building blocks of the body are assembled during the early stages of development. "This work will provide insights into the genetic and developmental causes of birth defects of the head and the face" said Prof Tam.

"This basic research lays the foundation for understanding the normal processes of healthy development and will ultimately feed into improved understanding and treatment of human illness" said the ARC panel that reviewed Prof Tam's research proposal, and that "Tam is a national treasure".

# \$100 million raised since 1958

More than 300 people gathered at Parliament House to celebrate 50 years of community fundraising in support of the Children's Medical Research Institute (CMRI).

Dedicated fundraising committees, business and individual supporters have together raised over \$100 million for research into illnesses affecting children and babies since the Institute was established in 1958.

Hundreds of committee members from Sydney, regional New South Wales and Canberra attended the Parliament House function. Other prominent supporters of CMRI who attended included music legend and Jamm for Genes® patron Glenn A Baker, television news host Ross Symonds, artist Nafisa Naomi and General Director of Channel 7 Sydney, Tim McDonald.

The CMRI fundraising committees, corporate and individual supporters have made considerable contributions to advancing the frontiers of medical research and are absolutely vital to CMRI's continued success.

The achievements in medical research in the past 50 years have been priceless. With continued support there is every reason to be confident that the pioneering work at institutes such as CMRI will have similar benefits to the health of our children and our children's children.



Ross Symonds and Jamm for Genes® Patron Glenn A Baker at Parliament House for the 50th Annual Meeting of Committees

*The photo in the "O" on the front cover shows a 148,000 X magnification of a nerve terminal. Mitochondria supply energy to the nerve terminal and here by chance they look like a smiley face. CMRI scientists study nerve terminals to better understand neurological conditions such as epilepsy.*

## Funding to improve gene therapy

Gene therapy is based on the idea that if a person is sick because of a mutated gene it may be possible to cure them by introducing a healthy copy of the gene.

Studies in gene therapy have been overshadowed by side effects associated with the treatments and there is a global effort to develop safer and more effective techniques. Some of the side effects have resulted from the therapeutic gene being inserted into the wrong place in the patient's own DNA.

"It may be possible to monitor patients more effectively following their gene therapy by knowing where the therapeutic gene is inserted" said Dr Samantha Ginn from the Gene Therapy Research Unit (GTRU)\* "or better yet, it may be possible to adjust the site of gene insertion by changing the conditions under which the therapeutic gene is prepared, prior to delivery to the patient."

To turn this idea into reality, researchers in the GTRU are collaborating with Dr Chialin Wei from the Genome Institute of Singapore, and Prof David Russell from the University of Washington, Seattle.

An International Science Linkages (ISL) Grant from the Federal Department of Innovation, Industry, Science and Research has just been awarded to the team and will provide \$487,688 over three years.

"Collaboration internationally and across disciplines underpins Australia's important contribution to the global research effort" Senator Kim Carr said of the ISL grant scheme, which is designed to boost the international reputation of Australian research.

\* The GTRU is a joint effort of the Children's Medical Research Institute and The Children's Hospital at Westmead.

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Rett syndrome is caused by a spontaneous genetic mutation that changes the levels of various proteins present in the brain. These altered protein levels are responsible for the symptoms of the syndrome.

In 2005, researchers at CMRI, in collaboration with Prof John Christodoulou from The Children's Hospital at Westmead, developed Australia's first mouse model of Rett syndrome, an essential step towards understanding the basic biology of the condition.

The Paul Newman Foundation grant enables CMRI to purchase equipment needed to analyse proteins in the brains of mice with Rett syndrome.

This research is expected to be completed within two years. After this time, further funding will be needed to allow CMRI scientists to search for treatments that restore these proteins to more normal levels, possibly reducing the symptoms of Rett syndrome.

# Jeans for Genes®

## Ricki-Lee Coulter Donates \$100,000 to Jeans for Genes

Ricki-Lee Coulter, the Face of Jeans for Genes 2008, singing sensation and presenter from Australian Idol was recently a contestant on the Channel 10 program "Are you smarter than a 5th Grader?" with Rove McManus. Ricki-Lee chose Jeans for Genes as her charity of choice and raised \$100,000 over two nights on the program. She said she had a great time on the show and was very excited about helping raise so much money for the Children's Medical Research Institute (CMRI). Ricki-Lee has now raised over \$105,000 for the organisation this year alone.

## Gift Wrap for a Cause

Jeans for Genes is once again gift wrapping at Christmas to raise funds for CMRI. Last year over 500 volunteers wrapped gifts for a donation at MYER, raising over \$40,000. This year we hope to once again raise some much needed funds at a number of locations across Sydney. If you would like to be a volunteer Genie please register online via the Jeans for Genes website [www.jeansforgenes.org.au](http://www.jeansforgenes.org.au)

## Jeans for Genes Golf Day 2008

Jeans for Genes and CMRI's Vaucluse-Double Bay Committee jointly held the second Jeans for Genes Golf Day in July at Concord Golf Course. The weather once again was wonderful and over \$40,000 was raised through sponsorship, a raffle and an auction.

## Jeans for Genes Day Update

Jeans for Genes Day 2008 has raised over \$3 million to date, with more funds coming into the office every day. Congratulations and thank you to everyone for their support this year.

If you are still to send in your money, please do so before Christmas. For further information please contact CMRI on 02 9687 2800.

## Kimberly-Clark Australia visit

Staff from Kimberly-Clark Australia (KCA), one of CMRI's major corporate partners, came to visit the Institute and see how their support is helping our research programs.

## For the Diary

### Friday August 7—Jeans for Genes Day 2009

*Volunteer Genies gift wrap for Mothers' Day 2008*



*Ricki-Lee Coulter has raised \$105,000 for Jeans for Genes*



*Phil Mayo, Gareth Shaw, John Glover and Alan Zammit at the Jeans for Genes Golf Day*



*Jeans for Genes team member Lynda Dave presents Fiona Sutton from KCA with a thank you plaque for their fabulous support of Jeans for Genes*





# Committee Power

The **Wagga Committee's annual Christmas Fair** was a tremendous success with almost all of Wagga doing their Christmas shopping for a great cause. The Wagga Committee also launched the museum exhibition "50 Years of Discovery" in collaboration with the Museum of the Riverina and CMRI.

The **Canberra Committee's market day** at Pialligo Estate Winery blossomed with stalls of every kind on a balmy Canberra day. Mummies, grannies, babies et al. poured in through the gates to catch some early Christmas gifts. Thank you Canberra Committee for organising such an enjoyable day in a delightful venue.

Glitz, glamour and lots of bling greeted the capacity crowd who turned up to hear the Sydney group "The Super Supremes" in the Quirindi Pavilion. The singing and dancing only stopped at the bewitching hour. Thank you **Quirindi Committee** and community for organising and supporting such a super event.

Strathfield Town Hall was packed to the rafters with keen minds getting top scores and enjoying a fabulous evening of trivia, lucky dips and silent auction prizes at the **Strathfield Committee Quiz Night**.

Over 500 expectant guests, all fashionably attired attended the long awaited **Hills Committee's Rosehill Gardens Race Day**. The races, raffles, lucky dips together with superb luncheon and perfect weather ensured there were only winners on the day.

The CMRI network spread far and wide when Jo Bevis and her team put on "The Biggest Girls Night of the Year" at the Altitude Bar in Dunsborough, WA. It was all girls, glamour and generosity that raised the gigantic sum of over \$10,000.

The **Becroft and Burbs Committees** provided the most delicious lunches for the 620 guests who toured the Galston Open Gardens in October.



# Other events

**Kimberly-Clark Australia (KCA)** have renewed their sponsorship of CMRI by donating \$450,000 over three years towards our research into congenital defects, Jeans for Genes and community awareness programs. CMRI Director Prof Roger Reddel presented KCANZ Managing Director Mark Wynne with a CMRI trophy in recognition of their 14 years of support.

## Good Will Week

CMRI has been selected as the charity partner for Good Will Week. Public Trustee NSW will donate \$1 to CMRI for every Will made with them before October 2009. Call 1300 14 24 34 or visit the website

[www.goodwillweek.com.au](http://www.goodwillweek.com.au) for more information.



Once again CMRI was the chosen charity of the **Muirfield Ladies' Charity Golf Day** and many thanks must go to the generosity of the committee and the players for their continued support. The **Becroft Committee's** home craft and cake stall added to the success of the day.

Top to bottom:

CMRI's Ruth Gordon with Wagga Committee President Gail Crozier (at the back) and past Presidents Peg Edols, Rhonda Walmsley and Inaugural President Libby Lamont at the launch of the "50 Years of Discovery" exhibition at the Museum of the Riverina;

Fiona Spencer and grand-daughter Sophia enjoying the Canberra Committee Market Day;

Quirindi Committee President Pam Lord and waitresses at "The Super Supremes" show;

Cassandra Stefanovic and Chris McGee at the Hills Committee Race Day;

Organisers Jo Bevis and Jo Richardson with MC Sara Williams (centre) at "The Biggest Girls Night of the Year";

KCA Research Fellow, Dr David Loebel, presents his research to KCA executives David Stillman, Peter Bennett, Glen Watts and Hourie Demirjian.