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Barring accidents, congenital abnormalities and genetic disorders are the leading causes of death in children aged 0-4, and cancer in children aged 4-14 ...

"... our mission is to discover cures for childhood diseases."
“A dream you dream alone is only a dream. A dream you dream together is reality.”

John Lennon
Children’s Medical Research Institute (CMRI) was Australia’s first dedicated paediatric medical research facility and has been helping to save the lives of children for over 57 years. We passionately believe that, together, we can beat childhood diseases.

CMRI arose from the dream of parents across Australia who wanted to stop children from suffering and who banded together to create the endowment fund that continues to fuel CMRI’s efforts today. We are an organisation which does research towards the prevention, treatment or cure of childhood diseases. All of our staff are passionate about our goals and want to make a difference. Throughout our administration and fundraising staff, our scientific support staff, and the many dedicated and talented research staff and students in our laboratories, you will find highly motivated people who believe in what they do. They know they are helping to create a healthier, brighter future for their children and yours.

But, despite our passion and efforts, none of our discoveries would happen without the support of people like you, whether you are a philanthropist, a member of our fundraising Committees, a Jeans for Genes supporter, a Great Cycle Challenge participant, or one of the many other members of the community who participate, donate, or organise events and encourage others to do the same. Without these connections to our community, and the unwavering support of that community, CMRI would be nothing more than a dream. Instead, we are working to make a better future, and that is reality.

In these pages, you will learn more about our unique research and what we are doing to find cures for childhood diseases, as well as how people like you are providing the support needed to help us reach our shared goals.

Welcome to our annual report for the calendar year, January through December, 2014.

There are more than 6000 genetic diseases
Our achievements are measured by the quality and impact of our research programs. 2014 was a year for major discoveries in each of our four core areas of research:

**Cancer**
- we identified a gene that causes inherited bone marrow failure
- we uncovered a key anti-cancer target for ALT cancers, some of the most aggressive types
- we’re learning how individual variations in our DNA increase or decrease our risk of cancer

**Embryology**
- we published evidence that exercise is beneficial for Rett syndrome
- we’ve developed a new understanding of RNA editing, a vital process in all cells, that has implications for embryo development and normal cell functions

**Genetics and Gene Therapy**
- we found a way to deliver our gene therapy treatments for metabolic liver diseases to the cells where the treatment is needed
- we’ve discovered genes that cause retinal disease, cataracts, and glaucoma

**Neurobiology**
- we’re gaining a better understanding of how the brain can rewire itself, which is important for learning and memory
- we’ve determined dynamin-inhibitors should be safe for treating cancer and epilepsy in humans
- we’ve shown that dynamin inhibitors can treat simian haemorrhagic fever—which means these compounds may ultimately prove useful for human Ebola infection

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**Highlights**

**OUR GOALS**

**Deliver gene therapy cures for rare genetic diseases in children**

**See that our epilepsy treatments help children (and adults) around the world**

**Develop new cures for kidney disease and for infectious diseases**

**Find new and better treatments for most types of cancer**
“Completion of Stage 1 of our redevelopment, which officially opened in August 2014, means we’ve almost doubled our research space and can accelerate our research efforts - a big step towards achieving our goals.”

Professor Roger Reddel
The official opening of Stage 1 of CMRI’s redevelopment on 29 August 2014 was a landmark event attended by The Hon Mike Baird, Premier of New South Wales and The Hon Jillian Skinner, NSW Minister for Health and Minister for Medical Research and Australian Health Minister Peter Dutton.

In the time since then, several of our major facilities—Bioinformatics, Biomedical Proteomics and The ACRF Centre for Kinomics—have moved into larger, better equipped laboratories.

In addition, another national resource for cancer research, The ACRF Telomere Analysis Centre (ATAC), has been set up in purpose-built rooms within the new building. By the time this report goes to print in May 2015, ATAC will have been officially opened by the new Minister for Medical Research, The Hon Pru Goward. Our redevelopment is already proving invaluable, enabling our researchers to access previously unavailable technology that can now speed up their work, in some cases doing in days what once took a year, or achieving things which were not even possible before.
As always, CMRI’s priority is to perform outstanding research that will improve children’s health. In 2014, our lead researchers, fundraisers, facility managers and operations teams came together to assess how best to achieve this goal in the coming years, reaffirming that genetic research is one of our major priorities. We considered the best ways to use recent advances in genome sequencing and related technologies to accelerate our progress, and how CMRI’s research capabilities can be integrated most effectively with the work of other medical research organisations.

We have begun to intensify our focus on genetics in the following ways. We are establishing a vector and genome engineering core facility which will be operational in 2015 and which will support all of our research efforts, especially in gene therapy. We are planning to increase our capacity to interpret the meaning of various genetic changes, to increase the ability of doctors to give the right advice for children with genetic diseases, and to make it possible to develop and refine potential treatments. We are also endeavouring to increase the capacity of our key facilities, like Bioinformatics and Proteomics, which will accelerate the rate of progress for all of our research teams.

The evidence that CMRI will be able to translate genetic information into cures for childhood diseases is found within the Research section of this annual report. The highlights from 2014 include work done together with colleagues at Children’s Hospital Westmead. Our Eye Genetics group not only continues to rapidly identify genes causing inherited eye diseases, but they are also making strides in understanding how these genes work and how treatments can be devised. Our cancer research teams showed that the TPP1 gene can cause inherited bone marrow failure. Our Gene Therapy Research Unit developed a critically important new technique for liver gene therapy and took the first steps towards clinical trials with international collaborators. In addition, we have research teams working towards treatments for epilepsy and brain cancer that are based on an entirely new principle of action. CMRI researchers published nearly 60 articles in international peer-reviewed journals in 2014, detailing their findings in cancer, embryology, neurobiology, and gene therapy. The progress we have made in each of these areas is due to the accumulated knowledge and expertise which your support has allowed us to build up over many years.

To maintain this momentum, and to continue developing the facilities and resources that will ensure that our discoveries increasingly make a difference to the lives of children, we need to move on to Stage 2 of our building redevelopment as soon as possible. This will be the major focus for our fundraising efforts. We are also increasing fundraising for the long-term support of our research programs. The Jeans for Genes campaign remains alive and well, still continuing to attract support in its 21st year. New initiatives will be introduced in 2015 to give Jeans for Genes a fresh approach. The Great Cycle Challenge broke its own record for the success of a new online peer-to-peer fundraising campaign, raising over $2 million in its second year, 2014, for our cancer research programs. Our very special Committees and their members continue to support us in our endeavours. They are our ambassadors in the community as well as being such committed fundraisers. We thank them and everyone who donated their time, money or creativity in support of CMRI.

In addition, we especially thank our Board and those who serve on our Committees, including the Audit and Risk, Finance and Investment, Nomination and Remuneration, Intellectual Property and the Building Foundation. The oversight of our Finance and Investment Committee was important for making 2014 such a success, with our net assets increasing by $10 million.

We are grateful for our loyal and enthusiastic staff, community supporters, and to our many national and international scientific collaborators who work with us towards our research goals. We continue to strengthen our organisational links with the University of Sydney, our Westmead Research Hub partners (Children’s Hospital at Westmead, Westmead Hospital, and Westmead Millennium Institute), and our paediatric research partners (Sydney Children’s Hospital Network and Children’s Cancer Institute). These networks enable the sharing of access to high-tech research equipment and research expertise.

CMRI’s work depends on collaboration. Research collaboration extends across Australia, New Zealand, Asia, the United States of America and Europe, and we collaborate closely with our community supporters across the country. Finding ways to prevent, treat and cure childhood diseases relies on everyone doing what they do best, whether it is making discoveries, translating those discoveries into treatments, or providing essential resources. Each link in the chain is vital, and on behalf of CMRI, we thank every one of you.

Sincerely,

Professors Frank Martin and Roger Reddel
May 2015
“We keep moving forward, opening new doors, and doing new things, because we’re curious and curiosity keeps leading us down new paths.”

Walt Disney
In no time, we have occupied about half of that building with new and expanded research facilities, as well as a larger space for our fundraising team. Plans are underway to introduce further research staff and another specialised facility before 2015 is over.

In line with this planned growth, the Institute’s revenue from its continuing operations grew to over $25 million in 2014, with approximately 75 cents of every dollar spent on operating expenses going to our research. Continuing good performance in financial markets has allowed us to almost cover our fundraising and administrative costs from our aggregated investment returns. In 2014, 97 cents out of every fundraising dollar raised went to our research programs and research support functions. (In 2013 – 100 cents out of every dollar went to research).

Our fundraising efforts contributed over $12 million to gross revenue in 2014 and continue to generate significant returns for CMRI, from big, branded events like the iconic Jeans for Genes Day or the new, app-based Great Cycle Challenge, to the traditional work of our Community Committees – tireless volunteers for over 50 years in many cases. Given our growth plans, we are looking to introduce some new events and campaigns in 2015, connecting with more people who are particularly interested in changing the future by supporting the valuable work of the Institute. Our fundraising costs represented 32% of the related fundraising revenue, (ignoring the building redevelopment funds). As mentioned above, these costs and our administrative costs were virtually covered by our investment income.

Our researchers maintained CMRI’s success with grant awards despite overall declines in national success rates, headlined by the Commonwealth’s main medical research program awarding grants at a rate of approximately 14% only. CMRI researchers have averaged success rates between 20 to 30% over the last few years, generating approximately $9 million in project and capital grants in 2014. This included a major award from the Australian Cancer Research Foundation to establish Australia’s first dedicated Telomere Analysis Centre, a facility containing very high resolution microscopy to explore a major feature of what goes wrong in all cancer cells.

As we completed our building and brought to account the final amount of capital grant income, our net assets have risen by $10 million to now stand at over $116 million, as at 31 December, 2014, most of which is invested in financial assets that will generate investment returns for use in the future operations of the Institute. Credit goes to our Board, which has carefully managed the growth of these investments over the years while continuing to fund our ongoing operations. We are fortunate to be able to use our investment earnings to supplement our other income sources and consistently fund long term research programs. We believe maintaining this spending balance is a major factor on the path to our research success.

Sincerely,

Ralph Mitchell
Chief Financial Officer & Company Secretary
May 2015

2014 was a notable year for CMRI, completing the first building stage of our planned and approved five stages of building development, for a total cost slightly under the $29m million budget that we set ourselves.
## Financial summary

### Profit and loss statement (in $ '000s)

<table>
<thead>
<tr>
<th></th>
<th>YTD Dec 2014</th>
<th>YTD Dec 2013</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Revenues</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Research</td>
<td>9,495</td>
<td>7,983</td>
</tr>
<tr>
<td>Fundraising</td>
<td>12,302</td>
<td>11,822</td>
</tr>
<tr>
<td>Investments</td>
<td>3,778</td>
<td>2,624</td>
</tr>
<tr>
<td>Building Redevelopment</td>
<td>7,350</td>
<td>13,532</td>
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<tr>
<td><strong>Total</strong></td>
<td><strong>32,925</strong></td>
<td><strong>35,961</strong></td>
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<tr>
<td><strong>Expense</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Research</td>
<td>19,035</td>
<td>18,248</td>
</tr>
<tr>
<td>Fundraising</td>
<td>4,038</td>
<td>3,818</td>
</tr>
<tr>
<td>Administration and facilities</td>
<td>2,343</td>
<td>2,244</td>
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<tr>
<td><strong>Total</strong></td>
<td><strong>25,416</strong></td>
<td><strong>24,310</strong></td>
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<td><strong>Surplus/(loss) before investment transactions</strong></td>
<td>7,509</td>
<td>11,651</td>
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<tr>
<td>Investment transactions, net</td>
<td>3,073</td>
<td>1,049</td>
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<tr>
<td><strong>Surplus/(loss) from continuing operations</strong></td>
<td>10,582</td>
<td>12,700</td>
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<tr>
<td>Other comprehensive income from Available-for-sale financial assets</td>
<td>(646)</td>
<td>7,106</td>
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<tr>
<td><strong>Total comprehensive income/(loss) for the period</strong></td>
<td><strong>9,936</strong></td>
<td><strong>19,806</strong></td>
</tr>
</tbody>
</table>

### Balance sheet (in $'000s)

<table>
<thead>
<tr>
<th></th>
<th>As at</th>
<th>31 Dec 2014</th>
<th>31 Dec 2013</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Assets</strong></td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>Current Assets, including cash &amp; term deposits</td>
<td>15,424</td>
<td>21,839</td>
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<tr>
<td>Other Financial Assets</td>
<td>69,922</td>
<td>68,420</td>
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<tr>
<td>Property, Plant and Equipment</td>
<td>43,280</td>
<td>30,454</td>
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<tr>
<td>Other Non-current Assets</td>
<td>475</td>
<td>808</td>
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<tr>
<td><strong>Total</strong></td>
<td></td>
<td><strong>129,101</strong></td>
<td><strong>121,521</strong></td>
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<tr>
<td><strong>Liabilities</strong></td>
<td></td>
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<tr>
<td>Current Liabilities**</td>
<td>11,987</td>
<td>14,393</td>
<td></td>
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<tr>
<td>Non-current liabilities</td>
<td>292</td>
<td>242</td>
<td></td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td></td>
<td><strong>12,279</strong></td>
<td><strong>14,635</strong></td>
</tr>
<tr>
<td><strong>Net Assets</strong></td>
<td></td>
<td><strong>116,822</strong></td>
<td><strong>106,886</strong></td>
</tr>
</tbody>
</table>

The above numbers have been extracted from the Audited Financial Statements of CMRI for the relevant periods. The full audited financial statements are available at www.cmri.org.au/About-Us/Annual-Reports-and-Financial-Statements
Sources of income, revenue and expenditure

Sources of income (excluding building redevelopment)

2014 – Total income: $28 million

- 22% Total investment returns
- 34% Grants for research
- 44% Fundraising

2013 – Total income: $31 million

- 35% Total investment returns
- 26% Grants for research
- 39% Fundraising

*Total Investment Return includes dividend distribution, profit on redemption and comprehensive income on available-for-sale financial assets

Sources of revenue (as per audited financial statements)

2014 – Total revenue: $33 million

- 11% Investments
- 29% Research
- 37% Fundraising
- 22% Building redevelopment

2013 – Total revenue: $36 million

- 7% Investments
- 22% Research
- 33% Fundraising
- 38% Building redevelopment

*Total revenue from Investments includes dividend distributions

Sources of expenditure (excluding redevelopment expenditure)

2014 – Total expenditure: $25 million

- 75% Research
- 16% Fundraising
- 9% Administration and facilities

2013 – Total expenditure: $24 million

- 75% Research
- 16% Fundraising
- 9% Administration and facilities
Research spend by disease

2014

Total research spend by disease*: $15.4 million

- 50% Cancer
- 18% Embryology
- 14% Gene Therapy
- 18% Neurobiology

2013

Total research spend by disease*: $15.2 million

- 51% Cancer
- 19% Embryology
- 12% Gene Therapy
- 18% Neurobiology

*does not include research facilities and research infrastructure like proteomics and bioinformatics
Sources of fundraising income

2014
Total fundraising income: $12.6 million

- 5% Trusts and foundations
- 6% Other events and campaigns
- 19% Jeans for Genes®
- 17% Great Cycle Challenge
- 10% Direct mail and regular giving
- 2% Building redevelopment donations
- 41% Bequests

2013
Total fundraising income: $12.9 million

- 5% Trusts and foundations
- 6% Other events and campaigns
- 19% Jeans for Genes®
- 9% Great Cycle Challenge
- 8% Direct mail and regular giving
- 8% Building redevelopment donations
- 44% Bequests

*Fundraising income includes building redevelopment donations.
1 in 20 children is born with a birth defect or genetic disease

CMRI researchers are facing these challenges head on. You’ll see that basic research (which means fundamental research) is a powerful approach to finding answers for a wide range of diseases. We have world-leading researchers with bright ideas, the right skills, and the energy and enthusiasm to get us where we need to go.

In this section you’ll get to meet our research teams and learn more details about their exciting work.
Sometimes the questions are complicated and the answers are simple.”

Dr. Seuss
Diseases impacted by our research:

- Most types of cancers, especially...
  - Glioblastoma
  - Osteosarcoma
  - Soft tissue sarcomas
  - Neuroblastoma

Image: ATRX in ALT cancer cells
The Cancer Research Unit investigates how cancer cells acquire the ability to multiply without limit – in other words, to become “immortal”. We are particularly interested in the role played by telomeres, which are DNA sequences that act as protective caps at the ends of chromosomes, and we are very fortunate that there are now three other telomere research groups at CMRI (led by Tracy Bryan, Hilda Pickett and Tony Cesare) who work with us on various aspects of telomere function.

Each time a normal cell divides, a small amount of telomere DNA is lost. This gradual telomere shortening functions as a “clock” which counts down and ultimately stops normal cells from multiplying further. Cancer cells evade this normal limit by adding back telomere DNA to overcome telomere shortening and thus become immortal. They do this using either an enzyme called telomerase or by using an Alternative Lengthening of Telomeres (ALT) mechanism. The overall aim of telomere research at CMRI is to find new cancer treatments that work by killing cancer cells that use either telomerase or ALT. The main emphasis of the Cancer Research Unit at present is on understanding ALT. Better understanding of this mechanism will enable us to develop better treatments against ALT cancers, which are some of the most aggressive types, including glioblastoma brain tumours, osteosarcomas, and several types of soft tissue sarcomas.

### 2014 Research achievements:

- Gained new understanding of how cancer cells switch on ALT. We showed that one of the normal functions of a gene (ATRX), which is very commonly found to be damaged in cancers that use ALT, is to keep ALT in check.
- Found that cancer cells which switch on ALT by losing ATRX gene function are more fragile than normal cells when exposed to various forms of stress. We are endeavouring to use this knowledge to design treatments that will kill ALT-positive cancers with minimal side effects on normal cells.
- Worked with the other CMRI telomere groups on a range of projects including molecular details of ALT, and a mutation that causes short telomeres. We organised a conference in Australia attended by international experts to discuss the best ways of treating patients with excessively short telomeres.
- Together with Dr Loretta Lau’s research group at Children’s Hospital Westmead, found that some childhood cancers don’t have either telomerase or ALT. This means that we will need to develop different treatment strategies for the relatively small group of patients who have this type of tumour.

### What’s next:

develop potential methods of specifically killing ALT cells.
Telomeres are the protective structures at the ends of our chromosomes. They are made up of repetitive DNA that is lost each time a cell reproduces, but this DNA can be added back using either an enzyme called telomerase or a process called ALT.

**Telomere length regulation** involves an intricate balance between lengthening and shortening processes, which ultimately determines the capacity of a cell to divide. Telomere lengthening is correlated with most types of cancer, while telomeres that are too short can cause aging and short telomere syndromes, characterised by bone marrow failure, organ failure or predisposition to rare types of cancer. We are investigating telomere trimming to regulate telomere length, how variants of the telomerase enzyme can confer cancer risk, and how variant telomere DNA sequences contribute to telomere function. This research will impact upon future treatment of cancers and short telomere syndromes.

**2014 Research achievements:**
- We identified variant DNA sequences in the telomeres of cancer cells that use ALT. We also identified several key proteins (ZNF827/NuRD complex) that interact with these sequences and can regulate the ALT mechanism. This was a major contribution to international ALT cancer research.
- We’ve gained mechanistic insight into how individual variations in our DNA (SNPs) predispose to breast and ovarian cancer.
- We contributed to a study that showed the telomere protein, TPP1, can cause a short telomere syndrome that causes aplastic anaemia.
- We are using bioinformatics to develop a machine learning algorithm that will allow computers to detect whether the DNA in a patient sample has short or long telomeres and whether a cancer uses ALT or telomerase. This will be important for personalised medicine approaches to cancer treatment.

**What’s next:**

develop new ways to identify and inhibit ALT cancer cell growth.

“We have been chipping away at ALT for many years, and now a very large chunk has fallen off into our hands. The results in this paper represent the single biggest advance regarding the molecular details of ALT that we have ever made at CMRI.”

Professor Reddel, about a recent paper from the Pickett group.
Diseases impacted by our research:

- all cancers
- short telomere syndromes, such as DKC and aplastic anaemia
Diseases impacted by our research:

- 85% of cancers
- Bone marrow failure and other short telomere syndromes
- Senescence (i.e. aging)
Our research focuses on one of the major factors in at least 85% of all cancers affecting children and adults: the molecule telomerase. Cancer cells use telomerase to keep growing uncontrollably; thus telomerase is a key target for future cancer treatments, which are predicted to have fewer side effects than current radiation and chemotherapy. We aim to understand the properties of telomerase in order to rationally design better anti-cancer treatments.

This includes studying the association of telomerase with telomere DNA, understanding how telomerase finds its way to the telomere, probing how the telomerase enzyme works and discovering its physical structure. In the long term, this knowledge will allow us to rationally design drugs against telomerase as potential anti-cancer therapeutics.

### 2014 Research achievements:

- We found that the TPP1 gene can cause a short telomere syndrome resulting in aplastic anaemia, where many patients die of bone marrow failure.

- We began screening more patients with aplastic anaemia for TPP1 mutations, in order to determine the cause of their disease.

- We have found many new proteins involved in bringing telomerase to the DNA, and thus we have many leads to investigate for inhibiting this process as a potential anti-cancer approach.

- We have found that a special DNA structure at telomeres, called a G-quadruplex, can modify how telomere length is maintained (using ALT or Telomerase), which could be important for most cancers.

- Through detailed study of the telomerase enzyme, we now understand which parts of the protein are most important—except for one area, called the C-terminus, which we are currently investigating.

- We determined a low-resolution structure of the telomerase enzyme, and we aim to have a higher resolution image of the structure in the near future. The clearer we can see the telomerase protein’s physical structure, the easier it will be to design potential anti-cancer drugs in future.

### What’s next:

What’s next is to gain a better understanding of telomerase structure and regulation to enable efficient anti-cancer drug design.
The Genome Integrity Group studies fundamental causes of cancer and aging. We seek to understand how our genome (the set of instructions guiding how our cells function) is protected, and when this protection fails, how a disease like cancer may result.

Children with mutations in genes important for genome health get cancers early in life and can develop rare syndromes. Much of our research focuses on telomeres, the protective caps at the ends of our chromosomes. We are studying how telomeres act normally during healthy aging to prevent cancer and how disruption in telomere function can result in changes to the genome that promote cancer. Recent work has centred on our discovery of intermediate-state telomeres. These are short “damaged” telomeres that are protected by a protein called TRF2. Prior to this discovery of an intermediate state, it was thought that chromosome ends adopted either a protected or unprotected state. What we now surmise is that the unique properties of the intermediate-state are what protect against cancer, and we are working on understanding exactly how this protection is achieved.

**2014 Research achievements:**

- We established a model system that will allow us to connect changes in telomere structure to biological outcomes, such as cancer.
- We are studying how the protein, ATM, is regulated in response to intermediate-state telomeres and how it communicates with the cell’s DNA repair machinery.
- We are also looking at how fully de-protected telomeres can cause a cell to arrest or stop growing. This arrest is believed to be important for preventing genetic changes that could lead to cancer.

**What’s next:**

make use of our model system to directly test the effects of changes in telomere protection state on cancer.

Children with short telomere syndromes are also prone to cancer.
Diseases impacted by our research:

- Cancer
- Aging
- Short-telomere syndromes

Image: PML bodies in a heart-shaped cell
Diseases Impacted by Our Research:

• All cancers, especially...
• Glioblastoma brain cancers

Image: super resolution image of proteins in a dividing cell
Our group studies normal cell division in order to understand what makes cancer cells grow out of control. We are also developing new anti-cancer treatments aimed at stopping cell division, especially in difficult to treat brain tumours, which are a leading cause of cancer-related deaths in young people.

**We operate** both a basic science research program and a translational drug discovery program. Despite the identification of many proteins involved in mitosis (the process by which cells divide and reproduce), the drivers of mitosis and how these proteins cooperate to complete mitosis is not fully understood. Cell division errors increase the potential of a cell becoming cancerous. Thus, understanding this basic biological process underpins our understanding of cancer, which can lead to the identification of targets for therapeutic anti-cancer drug design. We have already discovered that a subset of endocytic proteins are required for mitosis. Two of these (dynamin and clathrin) have been identified as anti-cancer drug targets, and these are the foundation of our drug development program, which is providing promising results.

### 2014 Research achievements:

- We have implemented a technique called super-resolution microscopy, which has allowed us to study cell cycle proteins more closely than ever before—at the level of a single molecule. This has opened up entirely new ways of understanding cell division.
- We are working on building a super-resolution microscope for CMRI, rather than relying on borrowed equipment located elsewhere. This home-built microscope should be more cost effective and function better than commercially available microscopes, helping our research and the research programs of other labs across CMRI.
- The improved understanding we now have of mitotic proteins will help our drug development program to better refine the compounds that work best to inhibit cell division in cancer cells.
- We are currently focusing on drugs that inhibit the clathrin protein, but will return to studying dynamin-inhibitors once we have the results of their effectiveness in a breast cancer model.
- We already have a class of compounds, called PITSTOP™, which we know inhibit clathrin. We have now developed a new class of drugs that bind clathrin differently. We believe these new drugs could have a synergistic effect with existing anti-mitotic drugs already in clinical trials. Clathrin drugs should be effective against all cancer types, but our research remains focused on the area of greatest need—brain cancer.

### What’s next:

continue to refine our lead compounds to find promising drugs that can one day be tested in clinical trials for brain cancer.
Cell Signalling Unit

Professor Phillip Robinson
BSc (Hons) PhD, NHMRC Senior Principal Research Fellow

The Cell Signalling Unit works to understand communication between nerve cells in the brain and between other cells in the body. Our work focuses on understanding an important factor in signalling, a protein called ‘dynamin’ and the other proteins involved in related roles inside cells. Basic research into how signals are sent between nerve cells and other cells in the body is aided by our developments in mass spectrometry technology and drug discovery, and in turn informs further advances in these technologies.

Our mass spectrometry technology allows analysis of phosphorylated proteins, key regulators in many biological processes, including nerve cell transmission. Our ability to study phosphorylated proteins using mass spectrometry has expanded exponentially, from 1-10 proteins at a time, to 200-300, and now to up to 5,000 at a time. Our approach is called functional phosphoproteomics, where we use mass spectrometry and other tools to constantly learn more about the activity in the synapses of nerve cells. Our research discoveries have led to a drug development program with potential to treat cancer, infectious diseases, epilepsy and other neurological disorders. This program has produced many exciting lead compounds, and we are in the process of the crucial but time-consuming process of identifying pre-clinical drug candidates to treat a range of diseases. This phase may take anywhere from 2 – 10 years due to the number of variables such as improving drug delivery, safety, and specificity. Crucially, we are on the path to revolutionising the treatment of conditions like epilepsy, where there is no cure and 1 in 3 patients are not helped by current medications.

2014 Research achievements:

• Other scientists have often claimed that a future dynamin therapy for humans would be risky. However we recently demonstrated that dynamin therapy for epilepsy and cancer is intrinsically safe for long term use. We showed that certain existing drugs used for mood disorders for extended periods of time in patients over the last 30 years also work by inhibiting the protein, dynamin, in the brain. This is a second and new mode of action to their known role in dopamine receptors. It means that the more effective versions of dynamin inhibitors currently being developed by CMRI and the University of Newcastle should be safe in humans, once the chemistry and formulation of these new compounds is perfected.

• We have demonstrated that dynamin inhibitors can also treat simian haemorrhagic fever—which means these compounds may ultimately prove useful for human infection with Ebola and other viruses.

• We published a series of high impact Methods papers aimed at teaching other researchers how to make and use dynamin inhibitors. These inhibitor compounds are now widely recognised in the broader scientific community for their research value and clinical potential.

• Our epilepsy drug development program has established 3 new classes of dynamin and clathrin inhibitors, providing us with new lead compounds for further development.

What’s next:

develop our epilepsy drugs to the point they can be used in clinical trials.
Diseases impacted by our research:

- Epilepsy and other neurological disorders
- Infectious diseases
- Kidney disease
- Cancer

Image: network of neurons
Diseases impacted by our research:

- Epilepsy
- Cancer
- Autism
- Alzheimer's
In the Synapse Proteomics Group, we are focused on answering fundamental questions such as how does the brain work? At the same time, we ask how a disease state, like epilepsy, can upset brain function. This fundamental knowledge is then applied to identify new potential drug targets. The genes we study code for proteins involved in neurotransmitter release (how chemical signals are sent from one brain cell to another), the shape of the synapse (the part of the brain cell that connects to another cell), or the ability of the synapse to adapt.

Developing treatments for diseases requires knowledge of the genes involved, the proteins they code for, and how to target those proteins with drugs to change the disease state. A better understanding of neurotransmission, for example, will help us determine what goes wrong in a range of diseases like epilepsy, autism and Alzheimer’s disease. It also tells us about normal learning and memory. We are also mapping the signalling networks between proteins inside the nerve cell and aim to add significantly to global efforts to model brain function. While finding treatments for adult-specific diseases, like Alzheimer’s or Parkinson’s, is not CMRI’s core mission, these discoveries are a serendipitous by-product of our work on childhood diseases and can potentially help many people suffering from neurodegenerative disorders.

2014 Research achievements:

• We were heavily involved in the design of the Proteomics facility in CMRI’s new building and have helped run it efficiently as a 24/7 operation.
• Developed expertise to further our studies of clathrin assembly, which is important for neurotransmission.
• We identified a new subdomain of the protein AP180, which functions in endocytosis, a key process in neurotransmission.
• Our work mapping signalling pathways for synaptic plasticity is ongoing, with new data continually leading us in new directions.
• We’ve begun studying the protein synuclein, which is involved in synapse plasticity and is well known to have a role in Parkinson’s disease.
• Due to a wealth of findings, we’ve begun many collaborative projects on pre-synaptic plasticity and pathways that intersect with disease to explore these results further.
• By the end of 2015 we should have a resource everyone can use to study their favourite protein in the brain.

What’s next:
use our new understanding of nerve signalling to find leads for potential treatment of autism, Parkinson’s and other diseases.

The number of children diagnosed with autism (ASD) doubled between 2002 and 2010, with 1 in 65 children now affected.
Embryology Unit

Unit Head: Professor Patrick Tam
BSc(Hons) MPhil PhD CBiol EurProBiol FAA FSB, FRS, Deputy Director CMRI, NHMRC Senior Principal Research Fellow

The Embryology Unit studies how development occurs in order to understand what goes wrong in birth defects. Current research focuses on the cellular and molecular mechanisms of body patterning during mouse development, in the context of gene activities that are important for the formation of the head and face, as well as in the development of the gut. Genes studied include Lhx1, Otx2, Twist1 and several CDC42-related Rho GTPases. The Rbm47 gene, which we are studying in gut development, turns out to be important for RNA editing.

In addition, we are improving our knowledge of cell differentiation, using a new type of stem cell we derived from mouse embryos. We have optimised the method to direct the differentiation of these cells into gut cells that then specifically become liver and pancreas cells. The goal of this research is to establish a method for treating inborn errors of metabolism using a combination of cell-based and gene therapies. This work is a collaboration with the Gene Therapy Unit in a joint effort of CMRI and the Children’s Hospital at Westmead.

2014 Research achievements:

- We are gaining an understanding of the process of cells transitioning between dispersed and tightly packed states, which is important for organ development. Such changes in cell state also occur during cancer metastasises, when cells from a solid tumour are disseminated, and then forms a solid tumour again in another part of the body.

- We have identified several key genes that are important for head and neck formation in the mouse and are working to understand the extensive gene regulatory networks they are a part of. We are also examining more closely the genes these key genes control, analogous to the cornerstones and the brick and mortar of a building respectively, to find out if when disrupted they may cause birth defects. Understanding these genes will enable us to alert clinicians of genes that may be relevant to human genetic diseases and which can be screened for errors in the specific gene in the clinic.

- We are interested in mouse embryo development, after the stage the embryo has been implanted and the body plan is being established. There are about 15,000 cells at this stage, and we know from fate testing that there is already high complexity and different fates set. We need to examine a smaller population of these cells to see how far they can differentiate. We could translate this knowledge to drive cell differentiation to generate clinically useful cells for implementing tissue therapies, modelling diseases, and testing the efficacy of drugs.

- A project in its infancy is the Rbm47 protein. We have identified this as a key factor that binds RNA in the gut tissues. We are setting up an innovative protocol to identify the RNA species bound by the RNA-binding protein and looking at the functional consequences of binding. We found binding mediates RNA editing that could produce an alternative form of the gene transcript, so two different proteins can be made from the same gene. The shorter protein is actually the working version.

- Our Rett Syndrome work shows that physical exercise helps slow the disease progression in the mouse. Exercise changes brain protein activity and gene expression. Following physical enrichment, the mouse responds better to anxiety and stress and shows changed behaviours. It is best if exercise is done early to delay development of symptoms. This information would be relevant to the care of Rett syndrome patients.

Infant and child mortality rates were more than halved between 1986 and 2010.
Diseases impacted by our research:

- Birth defects in early development, especially the gut and associated organs
- Head and face abnormalities including the Saethre-Chotzen syndrome
- Cleft lip and palate
- Genetic diseases of liver

In another study, we identified a biomarker to help with diagnosis of Rett-like syndromes.

Functional Genomics is critical for understanding what a gene does, and we are building the capacity to do the necessary work following the discovery of a mutant gene. We have already developed efficient genome editing capacity. This work will be significantly boosted by the establishment of the core facilities for vector & genome engineering and production of induced pluripotent stem cells, which are part of CMRI’s expansion plan. We have developed a fast track procedure for evaluating the impact of mutations using genome editing of mouse embryonic stem cells for embryological study, so we can determine more efficiently the effects of an erroneous gene on development.

What’s next:
develop our knowledge of cell differentiation for the benefit of disease modelling, gene therapy, and cell-based treatment for regenerative medicine.
Diseases impacted by our research:

- Genetic eye disorders, including...
- Retinal disease such as retinitis pigmentosa
- Childhood cataracts
- Glaucoma
- Rare syndromes with a visual component

Genetic retinal eye disease affects 1 in 3,000 Australians
Many of the genes that cause eye disease are not known. We aim to discover the underlying disease genes in order to develop new treatment strategies, in many cases where no current treatment exists. We use next-generation sequencing techniques and genomic investigations to pinpoint the disease genes.

The functions of the genes and of the proteins they encode are then determined using cell-based and animal studies. Only when we understand how a gene and its protein work, can we hope to find a cure. Disorders studied include: retinal diseases that affect the photoreceptor cells at the back of the eye; cataracts where there is clouding of the lens; glaucoma which can be associated with raised pressure in the eye; and conditions where there can be small or malformed eyes. Other genetic conditions are also studied where the causative disease genes are not yet known.

“What we need to do first is understand how the gene works, and then use this knowledge to develop a treatment…”
A/Prof Robyn Jamieson

2014 Research Achievements:

- We are developing a potential cell-based gene approach for investigating and treating retinal diseases.
- There are currently over 60 genes known to cause developmental eye disease. We established a next-generation sequencing strategy for detection of these, resulting in a significant improvement in disease gene identification for patients with these conditions. This is now being translated to clinical practice.
- There are over 120 genes known to cause genetic retinal diseases including retinitis pigmentosa, Leber congenital amaurosis, and cone dystrophies. We successfully used a next generation sequencing strategy for detection of disease genes in these disorders. This is now under translation to clinical practice.
- Use of next-generations sequencing has markedly improved clinical diagnosis in many of these genetic eye conditions, and this is especially so where the eye problem may be part of a larger syndrome, so this gives families early warning of additional health problems that will need to be addressed.
- We are still finding many new genes that cause eye diseases such as cataracts, glaucoma and retinal diseases.
- We've begun studying some eye diseases that are polygenic (many genes involved) with complex environmental factors, and are teasing out the roles of the individual factors. We need to make sense of the complexity in these diseases in order to better target therapies to particular genetic profiles.

What’s next:
- develop drug or gene-based treatments for retinal diseases.
The Gene Therapy Research Unit finds ways to correct genetic diseases in children. We are a joint initiative of Children's Medical Research Institute and The Children's Hospital at Westmead (CHW). Our primary goals are to develop more effective gene therapy methods and to translate basic research progress into improved health outcomes for children.

We are actively involved in the initiation of clinical trials for SCID-X1 and ornithine transcarbamylase deficiency (a urea cycle defect), which target the bone marrow and liver respectively and, in collaboration with the Children’s Cancer Research Unit at The Children's Hospital at Westmead, have a trial underway for children with brain tumours that are resistant to conventional therapy. The SCID-X1 and OTC trials involve collaborations with the Institute of Child Health in London. In SCID-X1, also known as “boy in the bubble disease” the aim is to use lentiviral vector technology to restore the ability of bone marrow cells to produce cells involved in immune system function, while in the OTC trial the aim is to restore the ability of the liver to convert highly toxic ammonia into urea. The MGMT trial aims to improve the resilience of bone marrow in children with brain tumours who are undergoing chemotherapy. This has the potential to allow them to withstand higher doses of chemotherapy with the aim of improving conventional treatment of their cancer.

In addition to clinical trials, we do extensive laboratory work. The primary focus of this pre-clinical research is the development of gene transfer and gene repair technology that can be safely used to treat diseases of the bone marrow and liver. While we focus on specific diseases, as mentioned above, the underlying technology has much broader implications and once proven safe and effective in selected diseases has the potential to be applied to many other troublesome diseases effecting the bone marrow and liver in infants, children and adults.

2014 Research achievements:

- We have established a strong collaboration with the Institute of Child Health in London to develop a clinical trial for urea cycle defects.
- We published a high-impact research paper in the journal Nature with collaborators at Stanford in the US, which highlights work underpinning our clinical trial plan using AAV vectors.
- We developed a new hybrid AAV/piggyback transposon gene transfer system that can cure neonatal lethal diseases (urea cycle defects and PFIC3 genetic liver disease) in mice.
- We are making progress in the combining of AAV-mediated genome editing methods with strategies for selective expansion of human hepatocytes with the aim of developing safer more effective therapies for a broad range of genetic and metabolic liver diseases.
- Working with Prof Patrick Tam, we have planned and won CMRI support for an exciting new initiative “The Vector and Genome Engineering Facility” (VGEF). In addition to providing a core service producing gene transfer vectors, genome engineering tools and engineered cell lines, the VGEF will also undertake discovery research seeking to improve and further develop these increasingly powerful technologies.

What’s next: implement an international clinical trial for the inherited liver disease treatment we developed.
Diseases impacted by our research:

- Many genetic diseases potentially treatable by gene therapy, especially...
- Bone marrow disorders
- Metabolic liver diseases

Image: skin fibroblast cells
Meet a researcher – Dr Annie Quan

What does your day look like?
Medical research is not a typical 9-5 job. The hours are dependent on the experimental work, some days can be 12 hours, but there is flexibility. I’m now a mum to a 2-year-old and working full-time. When I’m at work it is full concentration on the planned experiments. One of the most exciting aspects of being a medical researcher is that every day at work is different. We plan our experiments for each day, depending on the outcomes of the day before. We use a diverse set of tools and technologies to help answer our research questions which can be quite technically challenging.

What is involved in the research you do?
The research involves engineering the proteins of interest using recombinant DNA techniques and then studying the function of these proteins in neurons isolated from animal brain tissue. We can visualise where the protein is located in the neurons using high-resolution microscopes, and look for changes and modifications to the proteins with protein gels and mass spectrometers.

What are you trying to achieve?
The ultimate goal of my work is to increase our understanding of how brain cells sustain communication that underlies normal brain function. This communication process is called synaptic transmission. Abnormalities in synaptic transmission lead to many types of neurological diseases, including epilepsy, autism and Alzheimer’s disease. Thus, research in this area could lead to the identification of new therapeutic targets for these diseases.

What is one of your greatest research achievements and what impact has it had?
The greatest achievement to date is the discovery that syndapin-1 phosphorylation regulates its role in neurite growth rather than controlling neurotransmitter release. Why? During development and learning, neurons make billions of new connections with each other. This process involves the cells actively growing protrusions or ‘arms’ called neurites. The syndapin-1 protein regulates neurite growth by physically reshaping the brain cell through interactions with a network of other cell growth and shaping proteins. Thus the identification of a single modification with so much control over neuron growth means we now have a potential new target to aim at when developing therapeutic drugs. One day it may be possible to increase neurite growth and branching to help treat children with brain development medical conditions.

What type of research do you do?
My work is basic neuroscience research, examining the molecular mechanisms that control the signals for synaptic transmission, in specialised brain cells called neurons. Neurons are the basic unit in the brain that functions in learning and memory, and development. There are millions of neurons that make up the neural network in the brain. Inside these cells are proteins which control the release of chemical signals called neurotransmitters, contained inside round packages called synaptic vesicles, to sustain the process of synaptic transmission. I focus on studying the role of these proteins and the signalling pathways, with particular interest in the protein “syndapin-1”, that control synaptic transmission. I also have an interest in studying any abnormalities in this process that may cause the different types of neurological diseases.

Anything else to add?
The current medical research funding situation in Australia is dire, which means competition is enormous. Being a woman is even more difficult because of career disruptions with child birth and rearing responsibilities. These disruptions can have a negative impact on our competitiveness for funding. While it is taken into consideration during the application process, I would like to see more support for women in science, because we have a lot to contribute.
CMRI’s new Stage 1 tower provides purpose-built space for:

- Bioinformatics – Level 1
- ACRF Telomere Analysis Centre – Level 2
- Biomedical Proteomics – Level 6
- ACRF Centre for Kinomics – Level 6
In addition to accelerating research efforts within CMRI and the Westmead Hub with facilities such as the Bioinformatics Unit, CMRI provides important resources for scientists throughout Australia.

It operates CellBank Australia™, the only national repository of cell cultures, necessary for many fields of medical science. In addition, CMRI houses a major Biomedical Proteomics facility and two Australian firsts: the ACRF Centre for Kinomics, a joint venture with the University of Newcastle, which enables scientists to understand the master controls governing basic cell behaviour and develop new therapeutic drugs for a range of diseases; and the ACRF Telomere Analysis Centre, which will officially open in 2015.

“This facility offers hope to patients and families living with diseases where there is neither cure nor effective treatment. The research conducted has the potential to improve the future health of Australians and people around the world.”

Former Federal Minister for Health, Tanya Plibersek.
ACRF Telomere Analysis Centre

The ACRF Telomere Analysis Centre (ATAC) is supported by the Australian Cancer Research Foundation (ACRF) and the Ian Potter Foundation. The primary scientific focus of ATAC is centred on the study of telomeres, the structures at chromosome ends, and their roles in cell proliferation, cancer and ageing, with the secondary focus being to support a broad range of medical and biological research projects.

ATAC is located on Level 2 of CMRI’s Stage 1 expansion in a custom-built space designed to improve microscope stability and thermal regulation, especially for live cell imaging. Several new microscope systems, some available nowhere else in Australia, as well as image analysis facilities are available to facilitate the research programs of CMRI staff and collaborators.

ATAC is the brainchild of a unique group of telomere researchers from a broad range of scientific and medical backgrounds and skill sets including clinical haematology, medical oncology and laboratory-based research. This large consortium of telomere researchers will utilise cutting-edge equipment and specialised research techniques in this internationally unique Centre for collaborative telomere research.

The equipment consists of state-of-the-art microscopes and analysis stations that will allow researchers to adopt the latest research methods including:

- Tracking DNA movements in living cells over time
- Examining and measuring telomere DNA structures in minute detail
- Examining thousands of cells at a time using automated scanning

ATAC will focus on four key components of telomere-related research, including:

1. Telomere Length Analysis. ATAC will support collaborative epidemiological and clinical studies throughout Australia, in which telomere length is used as a biomarker.

2. Automated Metaphase Chromosome Scanning. CMRI has pioneered the use of automated scanning for metaphase cells in telomere research, and the ATAC equipment will increase capacity and add the latest technology in this area.

3. High-Resolution Fluorescence Microscopy. The length of individual telomeres, telomere structure, and telomere-interacting proteins are all critically important for telomere function and research. The microscope technology that will be available at ATAC will greatly enhance the researchers’ ability to study these characteristics at telomeres.

4. Live Cell Imaging. Telomere structure and function changes dynamically during the cell cycle. Technology at ATAC will put our research groups at the forefront of this field by providing researchers with the ability to study telomere dynamics in live cells, which will speed up progress in this area of research.
Bioinformatics is the application of information technology to the study of biology and medicine. Modern molecular biology research uses a variety of techniques such as genomics (large scale sequencing of DNA) and proteomics (large scale identification and characterization of proteins by mass spectrometry) that generate vast volumes of data. Sophisticated computational techniques are needed in order for the data to be correctly acquired, stored, managed, visualized, analysed, and interpreted. Bioinformatics is essential to modern health and medical research and needs to be an integral component of every biological research group.

The CMRI Bioinformatics Unit is a research, training and service facility dedicated to meeting the bioinformatics and computational biology needs of CMRI staff and students. Under the leadership of Associate Professor Jonathan Arthur, the unit:

- coordinates bioinformatics activity within CMRI
- provides a professional support and development network for bioinformatics staff embedded in CMRI research groups
- collaborates with CMRI research groups on research projects with bioinformatics components
- provides training and education for laboratory-based research staff and students to develop computational biology skills
- provides services and support in bioinformatics and bioinformatics infrastructure through a dedicated bioinformatics facility

CMRI Bioinformatics will also play a key role in developing and establishing bioinformatics capability within the Westmead Research Hub and is working collaboratively with other bioinformatics initiatives throughout NSW and Australia.
Human or animal cells grown in culture (cell lines) are an integral part of health and medical research. They serve as a model system for the study of cancer and many other diseases and are used in the discovery of new treatments. Without proper handling, cell lines can become contaminated, which can negatively impact any research conducted using those cell lines. For this reason it is critical for Australian researchers to have easy access to high quality, validated cell lines for their research.

CellBank Australia™

CellBank is Australia’s only national cell line repository, providing Australian scientists with ready access to validated cell lines and associated quality control services. CellBank Australia collects novel cell lines, developed by Australian researchers, submits these cell lines to rigorous testing to confirm their integrity; and then distributes the cell lines to researchers throughout the world.

CellBank Australia also distributes, throughout Australia and New Zealand, more than 700 of the most commonly requested cell lines from the European Collection of Cell Cultures (ECACC) at Public Health England, a major international cell line repository based in the UK. This ensures that a wide range of authenticated cell lines are readily available for use by Australian scientists.

In addition, CellBank Australia offers a variety of cell line-related services including STR profiling, Mycoplasma testing, Culture and Return, and Secure Storage. It is also part of CellBank’s goals to create awareness of the issues surrounding cell line integrity and contamination and to assist with the education of the Australian scientific community about these matters.

CellBank Australia has International Depository Authority status, under the Budapest Treaty on the International Recognition of the Deposit of Microorganisms for the Purposes of Patent Procedure. This means CellBank Australia is the only site in Australia that can provide secure storage for human and animal cell lines and hybridomas that are the subjects of patent applications.

CellBank Australia receives funding from the Cancer Institute NSW and was also the recipient of a National Health and Medical Research Council Enabling Grant from 2005 to 2010. It was established in 2005 with support from Cure Cancer Australia Foundation and a major donation by Lady Mary Fairfax via the National Breast Cancer Foundation.

Visit cellbankaustralia.com.au
Biomedical Proteomics at CMRI is a collaborative research platform providing high-throughput approaches to study the proteins in our cells and tissues. Understanding the structure and function of each protein in the human body and the complexities of their interactions is critical for the development of effective diagnostic and disease management tools in the future. The Facility supports scientists at CMRI and the Westmead Research Hub by offering not only access to sophisticated equipment and methodology but also expert advice in assessing the feasibility of new research projects and programs and developing strategies to implement them.

CMRI took an early lead in proteomics in 1998, and has been retaining and nurturing talent in this area for the past 16 years. With the completion of Stage 1 of CMRI’s building redevelopment in mid-2014, Biomedical Proteomics and the ACRF Centre for Kinomics were relocated to a large, modern, custom-designed space. This has resulted in vastly increased capacity and capability. The expansion was vital for the CMRI team to continue their ground breaking discoveries and to maintain their reputation as leaders in the field.

Biomedical Proteomics currently houses a total of seven advanced liquid chromatography-mass spectrometry (LC-MS) systems. Four were purchased with generous funding from the Australian Cancer Research Foundation (ACRF) and the Ramaciotti Foundations to equip the new ACRF Centre for Kinomics (ACRF-CFK). Major funding by the Cancer Institute NSW, the Australian Research Council Linkage Infrastructure, Equipment and Facilities scheme, and the Ian Potter Foundation provided two additional LC-MS systems.

This massive investment in new technologies has enabled the rapid progress of large-scale, long-term studies with publication of several papers in 2014. In addition, in partnership with SCIEX, several research and development projects were initiated that have opened up opportunities for scaling up joint research efforts. The Facility has also established an ongoing series of seminars, workshops and other educational and training opportunities for staff and students.

“The Research Hotel” was introduced with the stage 1 redevelopment. This means we have capacity for up to 20 visiting researchers to stay and complete their proteomics studies at CMRI. This includes access to the wet lab, instrument space, data lab and conference room, all while receiving expert research and technical advice from CMRI staff. Our goals are to maximize use of existing capacity, maintain the highest standards and international reputation, while enabling researchers from anywhere in Australia to benefit from our facilities.
ACRF Centre for Kinomics™

The Australian Cancer Research Foundation (ACRF) Centre for Kinomics (supported by the Ramaciotti Foundations) officially opened on 23 September 2012.

The ACRF-CFK — a joint venture of CMRI (Biomedical Proteomics) and the University of Newcastle (UoN) (Medicinal Chemistry) — is an Australian-first that provides an entirely new chemical biology approach to the understanding of cancer therapeutic drugs and ways to improve them.

This significant, non-commercial initiative builds upon demonstrated research excellence, leadership and successful collaborations between scientists from CMRI and UoN. The ACRF-CFK is equipped with state-of-the-art instrumentation, thanks to generous funding from the Australian Cancer Research Foundation (ACRF) and the Ramaciotti Foundations.

The Centre’s main focus at present is to facilitate a smooth transition to a fully functional, large scale national research platform, providing technologies and support for the discovery process that underpins progress in many of CMRI’s research areas. The priority is to ensure that these facilities realize their full potential to enhance Australian medical science and drug design and discovery.

Through the development of Kinomics technology and through its collaborations with research teams across Australia, the Centre will enable a better understanding of current therapies and their unwanted side effects. More importantly, the Centre will aid the development of new drugs for a broad spectrum of human diseases, many of which are currently without any effective treatment.
Children’s Medical Research Institute (CMRI) is an independent organisation that has no guarantee of government funding and competes for grant funds from many sources. However, CMRI does not work in isolation. Our researchers have collaborations with over a hundred different research groups in various institutions and universities in Australia and New Zealand, North America, Europe, and Asia (See the Addendum to the 2014 Annual Report for the full list).

In addition, CMRI is affiliated with the University of Sydney and many of our researchers have conjoint appointments as lecturers and professors and also supervise advanced degree students of the University. Furthermore, CMRI is involved in several large, formal, partnerships between institutions, universities, hospitals, researchers, and clinicians. Some of these partnerships are listed below:

**Sydney Health Partners.** CMRI is a member of Sydney Health Partners, which recently won recognition from the NHMRC as one of only four Advanced Health and Research Translation Centres in Australia. This means the centre was judged to be on par with the world’s best research and translation centres by a panel of international experts.

**Paediatric Research Initiative.** In February 2015, the NSW Minister for Health and Minister for Medical Research, Jillian Skinner, announced $15m for this initiative, which has not yet been officially launched. It is an innovative translational research partnership of The Sydney Children’s Hospitals Network (Randwick and Westmead), Children’s Cancer Institute (CCI), and CMRI that covers nearly all paediatric medical research in NSW.

**Kids Cancer Alliance (KCA).** CMRI is a founding member of KCA, an initiative of the Cancer Institute NSW (NSW Ministry of Health). KCA aims to take new discoveries from their development in the laboratory through to their translation in the clinic. It encompasses three child cancer-focused medical research institutes (Children’s Medical Research Institute, Children’s Cancer Institute, and the Kids Research Institute) and the three clinical care centres in NSW (Sydney Children’s Hospital Network at Randwick and Westmead, and John Hunter Children’s Hospital in Newcastle), in partnership with two major universities who support their research efforts – the University of New South Wales and the University of Sydney. Together, these hospitals treat more than 40% of Australia’s 600 newly diagnosed child cancer patients each year. KCA’s vision is to bring doctors and scientists closer together to accelerate discovery and its application to improve the care of, and outcomes for, children with cancer. Learn more at [www.kca.org.au](http://www.kca.org.au).  

**Westmead Research Hub.** The Westmead Research Hub is a formal coalition that includes CMRI, Western Sydney Local Health District, Sydney Children’s Hospital at Westmead, Westmead Millennium Institute, and the University of Sydney. Scientists across the Westmead Hub collaborate, sharing knowledge and expertise. The Hub has also established a network of Core Technology Facilities, where the acquisition, operation and use of highly specialised equipment is shared between the partners. CMRI is the host for a major Biomedical Proteomics Facility which incorporates the ACRF Centre for Kinomics (itself a major collaboration with the University of Newcastle). For more on this see [www.wrh.org.au](http://www.wrh.org.au) and [www.cmri.org.au/ACRF-CFK](http://www.cmri.org.au/ACRF-CFK).

**A Vision for the larger Westmead Precinct.** Western Sydney is the fastest growing economy in Australia and this is being recognised with major investments into the Westmead Precinct and surrounding areas. Over $1 billion is earmarked by the NSW government for development of the Westmead Hospital and the Children’s Hospital; the University of Sydney has announced plans for a full second campus in the Precinct; infrastructure will be improved by a $2 billion investment in light rail through Parramatta and larger amounts for the new Badgerys Creek airport plans. In addition to the Westmead Research Hub members, there are many other government and private entities participating in the Precinct’s growth, such as Westmead Private Hospital, Cumberland Hospital, and NSW Pathology West. The vision is for Westmead to be the largest and most innovative Health and Medical Research precinct in the Southern Hemisphere, and CMRI will be looking to play a significant role in these plans.
Fundraising

CMRI was established from grass roots community support in 1958 and still relies heavily on the support of individuals and community groups to achieve its long term research goals. Two-thirds of the Institute’s revenue comes from private fundraising sources, including the Jeans for Genes® campaign, community fundraising, bequests, direct marketing and a long-established investment fund.

The following pages highlight the achievements of CMRI’s fundraising programs in 2014 and acknowledge supporters who gave generously of their time and money to help create a healthier, brighter future for all children.

Some fundraising successes:

More than 2000 schools participated in Jeans for Genes

Jeans for Genes and Great Cycle Challenge each raised over $2m

The Gala Dinner alone raised $200,000

“It takes a whole village to raise a child”

An African Proverb
Jeans for Genes® campaign

Jeans for Genes® is one of Australia’s most recognized and loved charity campaigns and is a major fundraiser for Children’s Medical Research Institute.

2014 was the 21st Anniversary of Jeans for Genes, an occasion celebrated with enormous energy and enthusiasm across the country. Each year, the campaign pulls together Australians from all walks of life in a united show of support for the work CMRI does and this year was no different. Over 8,000 people got behind the cause by selling merchandise or collecting donations at work, clubs and social occasions which in turn directly touched more than a million people.

2,357 schools raised well over $500,000 and took our message to an estimated 1.5 million children ensuring our campaign has a strong and bright future, thanks to the ongoing support we secure throughout a child’s school years and beyond.

At 45 locations across the country, Jeans for Genes was represented by an army of 1,200 volunteers, including our wonderful CMRI committees, who raised money from dawn till dusk at train stations, shopping centres and street corners. Thanks goes to some of the country’s most recognisable retail chains, including Big W, Outback Steakhouse, HCF, Lowes, Crazy Clark, Sam’s Warehouse, Newcastle Permanent, Live Life Pharmacy, and Jeanswest who contributed their support across 850 stores nationally. Our media partners kindly donated $3M in advertising support, and we were proudly promoted by our ambassador – Channel 7 celebrity, Rachel Finch – on television, radio and in magazines. To top it all off, Staples chose Jeans for Genes as one of three charity recipients for its 2 million and change program.

By the end of the campaign, over $2 million was raised and the groundwork for a fantastic 2015 was established. In fact, well over 1,000 supporters already registered their details even before the 2015 campaign officially opened, so we’re looking forward to a massive year ahead! 2015 heralds a new era for Jeans for Genes as we continue to create innovative ways for the public to support our cause.
Finding cures for childhood diseases

Proudly supporting Children’s Medical Research Institute

Celebrating 21 years of Jeans for Genes

Or donate online jeansforgenes.org.au

Look for our merchandise and please give generously!

21 Years Young

Jeans for Genes

CHILDREN’S MEDICAL RESEARCH INSTITUTE – Annual Report 2014
**Gala Dinner – NY style!**

What a way to celebrate the Jeans for Genes' 21st birthday, in New York style. The 2014 annual Jeans for Genes Gala Dinner on Saturday, 23 August was nothing short of spectacular. The night themed with New York style, with guests truly getting into a New York state of mind.

Each year we are lucky enough to be generously supported by artists, sponsors, volunteers and special guests and this year was no exception. The lovely Chris Bath once again gave her time to be our fabulous Master of Ceremonies for the evening.

The night’s entertainment was sophisticated and stylish, with a performance from the Village Performing Arts Group as well as some of Australia’s best talent including Greg Gould, a finalist in Australia’s Got Talent, Danni Da Ros AKA Pocket Rocket Diva who is straight from The Voice Australia, Matt Lee, winner of a Helpmann Award for Best Actor in a Musical and Michelle Barr who had worked alongside Robbie Williams and David Campbell.

It was Adam Spencer’s first year as Auctioneer and he did a brilliant job with the right amount of entertainment and enthusiasm to get some healthy bidding going in the room! We are thrilled at the result with just short of $230,000 raised from the evening which will go directly towards vital research projects.

We were all touched by Chris Hart sharing his personal story about the ordeal that his family has endured over the past few years. It was great to see his son Ryan there with his family and doing so well, now nine months since his last treatment and everything is looking positive. Their story reminds us of the need for medical research.

Director of Children’s Medical Research Institute, Professor Roger Reddel, highlighted some of our greatest achievements, and talked about our goal to accelerate the rate at which we can bring treatments and cures to children everywhere.

Thanks once again to our major sponsors: Delta Airways, Burwood Press, Decorative Events, Woolworths Limited, Sofitel Luxury Hotels and Dan Murphy’s.

A special thanks to our generous volunteer Gala Dinner Event Committee Members, Patsy Cadell, Glenn A Baker, John Glover, Rod Glover, Patti Payne and Jacquie Randall. Each year they work tirelessly behind the scenes to make the event such great success.

None of this would be possible without the kindness, compassion and ongoing commitment of the guests. Medical research is the only way that we can tackle the big questions on life-threatening diseases and find answers sooner.

Thanks to all that were involved on the night and for once again sharing our vision and bringing greater hope for the future to families and children, you really made our 21st Jeans for Genes Gala Dinner a most memorable night.
The Great Cycle Challenge is a fundraising initiative launched in 2013. Riders all across Australia took on a personal cycling challenge to pedal throughout October and fight kids’ cancer. Participants logged their kilometres via a GPS enabled app and used online fundraising pages to seek sponsorship. 6,800 riders participated in 2014, collectively raising more than $2 million for CMRI.

Levi is 10 years old, he lives with a brain tumour and fights cancer every day of his life. He’s undergone round after round of chemotherapy to stabilise his brain tumour and suffers from extreme fatigue and exhaustion.

But this is no ordinary young man, because Levi set himself a goal to ride 400 km on his spin bike as part of the 2014 Great Cycle Challenge. Levi raised an incredible $3,155 for CMRI’s cancer research programs because “I don’t want any more of my friends in hospital to die.”

Levi joined 6,800 participants who set a ride goal and fundraised all around Australia throughout October. The event raised an amazing $2,123,000 – nearly double what the event raised in 2013.

Special thanks to our GCC Partners in 2014, including the firm Ridley, Jackson Teece and Lahey, and to Executive Channel.

The Great Cycle Challenge will be held again in October 2015. To find out more about Great Cycle Challenge and to get involved visit www.greatcyclechallenge.com.au.
Community fundraising

CMRI relies on the devoted support of community groups and individuals, who host a wide range of fundraising activities and events, such as the annual Earle Page College Coast Run and Earle Page College Fashion Show. We are ever grateful for their energy and enthusiasm and the funds they raise.

Our amazing CMRI committees are the foundation of our community fundraising and public awareness efforts. They are a model for community involvement, and each year their tireless work benefits all of our medical research programs. A list of their fundraising achievements can be found in the online addendum to this report. Some highlights from 2014, were:

• Vaucluse Committee
  Bill Waugh Memorial Cup Tennis Day
• Canberra Committee’s Annual Luncheon
• Hills Committee’s Mothers’ Luncheon
• Hills Committee’s Race Day
• Strathfield Committee’s June Gala Dinner
• Wagga Wagga Committee’s Christmas Fair
• Gerringong Committee’s High Tea
• Gerringong’s Famous Annual Quilt Show
• Gosford Committee’s Christmas Garden Party
• Port Hacking Luncheon and Fashion Show and Jazz for Genes
• Taree Baby Show
• Melbourne Cup luncheons, sweeps and raffles held by Kangaroo Valley, Racquet and Northern Beaches committees
• Mudgee High Tea
• Quirindi Luncheon
• Golf Days – hosted by Northern Beaches, Strathfield, Port Hacking Committees
• Card Days – Racquet, Northern Beaches Committees
• Quiz Night – Strathfield, Gosford Committee
• Discovery Day catering – Beecroft Committee
• Maroota Fashion Shows
• Ku-ring-gai Film Night

Other wonderful community supporters also include: Trivett Class BMW Parramatta, with 12 years of support for the Golf Day; Rotary Club of Cessnock Wine Country; and Treasury of Craft, with 22 years of dedicated fundraising via craft stalls; and the Eastwood and the Bankstown orchid societies for many years of support.

New supporters:
Younis and Co Walkathon

Younis and Co came on board as supporters of CMRI in 2014, when they hosted their first Younis and Co staff walkathon. The team walked from their Western Sydney office in Parramatta, all the way to their office in Sydney CBD – a total of 24kms! The team rallied the support of their clients and raised $30,000 for CMRI.

Jeans for Genes
Fun Run and Walk 2014

On Friday 29th of August, our loyal supporters at Woolworths made their annual Jeans for Genes Fun Run and Walkathon the biggest year ever and raised more than $50,000.

More than 100 participants from around the Norwest Business Park braved the rain and cold weather to walk or run the 5 km lunchtime race and support CMRI’s research.

Special thanks to our guest, Michelle Bridges, who ran the warm up even though she was unwell, and to our friends at Woolworths who hosted the event, donated countless raffle prizes and put on a lovely lunch for our hungry runners.

It was a fantastic day, and we look forward to doing it all again in 2015.

For more information about the Woolworths Fun Run and Walkathon in 2015, visit fundraising.cmri.org.au
**Bequests**

One of the most life-changing ways you can support CMRI is to leave a bequest in your will; it's a lasting legacy that will benefit future generations of children. Bequests are a vital source of income for CMRI, and we are always humbled by the generosity of our supporters who have left us a gift in their will. Here's what Nigel Armstrong has to say. He's an everyday person who plans to leave CMRI a gift in his will:

"I was a Postmaster for 38 years, now retired. An everyday person. I have no medical or academic background, but that doesn't stop me from being able to help.

"My wife passed away from breast cancer in 2003. She'd lost about five people on her side to cancer, and on my side I've lost a brother. I have another brother with leukaemia. I realised genetics must be important. I got in touch with Children's Medical Research Institute and learned about the work they're doing on DNA and telomeres. Telomerase is a fantastic line of research. From what I’ve learned, if we can stop telomerase in cancers we can stop them from proliferating. It's important to find better treatments for many types of cancer that affect everyone. Everyone knows someone affected.

"After my wife died, I wanted to do something worthwhile.

"When I heard about Sir Lorimer Dods, and how he set up the funding for CMRI for the long term, I thought it was a good thing. Research takes 15-20 years or longer, and you can’t rely on governments that change to keep it going. The way CMRI is set up, you can be sure a line of research will be followed through to its finality … CMRI’s research has the potential to help everyone, children and adults."

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**Major supporters and partners**

Children's Medical Research Institute and Jeans for Genes are supported by a wide range of individuals, businesses, trusts and foundations. A full listing of key supporters is provided in the addendum to this report available on our website.

We especially thank our major supporters, including Mr James Fairfax, Australian Cancer Research Foundation, YuHu Group, Memocorp, Ian Potter Foundation, Beryl Raymer, Mrs Joan M Barnet, Woolworths Support Office Bella Vista, Franklins, and J.J. Richards & Sons.

We also acknowledge the long term support of the Judith Hyam Memorial Trust Fund for Cancer Research whose generosity has enabled the continuance of two named positions in our Cancer Research Unit. The work of other young PhD students and scientists at CMRI is supported by scholarships from a number of generous sources: Yass Memorial Scholarship, Douglas and Lola Douglas Scholarship, Denise Higgins Scholarship, Rosemary Raymer Scholarship, Sir Norman Gregg Fellowship, and the Star Alliance Travel Scholarship.

Our corporate partners are important to our fundraising efforts, and we are fortunate to have long standing relationships with a number of companies. Some of our corporate supporters provide a combination of pro bono services and financial sponsorship. Particular thanks goes to Burwood Press, Technology One, Addisons Lawyers, Allen & O’Linklaters, Star Alliance, Cherry Media, Vinva Asset Management and Sciex for their generous support throughout 2014.

In the year ahead, we will continue to develop new and existing partnerships, and look forward to even bigger achievements together in years to come.
“How brilliant, how absolutely brilliant,” Meg Martyr said about the official opening of CMRI’s new seven-storey research tower extension on 22 August, 2014.

Meg recently lost her 15-year-old son, Hamish, to brain cancer and her daughter, Sophie, is now battling the disease but in remission. A floor in the new tower was purpose-built to house cutting edge equipment to speed up research into brain cancer and other cancers, and that is only one of many advancements the new structure will enable.

The Stage 1 tower is the first of a proposed five-stage rebuild of the Institute that is needed to support our expanding research efforts, accommodate greater student intake, provide improved infrastructure for specialised technologies, and to allow space for our growing national facilities. This will lead to better and faster translation into health outcomes and commercial opportunities.

Stage 1 was opened with NSW Premier Mike Baird, NSW Minister for Health and Medical Research Jillian Skinner, and Australian Health Minister Peter Dutton presiding over the official ceremony. The NSW Government contributed $20 million to fund the expansion. CMRI’s redevelopment is part of ongoing development plans for the whole Westmead precinct, all of which will create a world-leading centre for health and medical research.

CMRI Building Foundation Ambassador, Australian fashion designer Leona Edmiston, said, “Children’s Medical Research Institute has technology and expertise that is helping scientists around Australia find cures for genetic diseases, epilepsy, and birth defects. But they can do more to help others if we help them.”

The Building Foundation of Children’s Medical Research Institute was founded to ensure the successful redevelopment of CMRI facilities at Westmead, as well as strengthen relationships with members of the community - both foreign and domestic - to encourage fundraising on behalf of the building project.

The Foundation was officially launched on 14th November 2014, and the event was a great success, raising $207,900 on the night, enough to fund the full construction of a laboratory, thanks to the generosity of Mr Huang and YuHu Group, Vanessa Tay and Memocorp, Professor Reddel of CMRI, Rockpool Bar and Grill, as well as other big-hearted Building Foundation members.

The Foundation’s Executive includes its President, Mrs Carolyn Forster, its Honorary Chairman, Mr Xiangmo Huang, and members Mr Bruce Fink, Mr Albert Wong, Ms Virginia Judge, and Professor Frank Martin.

The second stage, costed at $60 million, will commence as soon as further funding can be jointly committed from government and the community. The second stage is vital to reaping the full benefits of stage 1, as it will house key facilities, such as those for functional genomics. Equally important, it has the additional laboratory space needed to strengthen and accelerate our existing research output, and take our discoveries to the next level for the benefit of children and their families.

Supporters contributing above $5,000 to the redevelopment are eligible to become members of the CMRI Building Foundation – entitling them to official acknowledgement, invitations to Building Foundation events and other great opportunities.

If you would like to contribute to our redevelopment please visit www.cmri.org.au/redevelopment, or contact Virginia Judge, Head of Strategic Partnerships on (02) 8865 2800.
"The future belongs to those who prepare for it today."

Malcolm X
CMRI is an independent research institute based in Westmead, NSW. The Institute employs approximately 160 people, including 120 full-time scientists and PhD students, as well as operational and administrative support staff and a team of fundraisers. CMRI is a public company, limited by guarantee and a registered charity. The organisational structure of CMRI reflects its corporate governance and areas of responsibility.

A researcher needs **12 years** of post-graduate and post-doctoral training.
The organisation

CMRI Board of Directors

Intellectual Property Committee  Audit & Risk Committee  Director  Building Foundation  Finance & Investment Committee  Nominations & Remuneration Committee

Operations  Research  Specialised facilities  Executive  Fundraising

- Lab Management
- Bio Resources
- Building Facilities
- Information & Communications Technology
- Cancer Research
- Cell Biology
- Cell Cycle
- Embryology
- Gene Therapy
- Cell Signalling
- Protein Biochemistry
- Eye Genetics
- Genome Integrity
- Telomere Length Regulation
- CellBank Australia™
- Bioinformatics
- Biomedical Proteomics Facility
- ACRF Centre for Kinomics™
- ACRF Telomere Analysis Centre
- Director’s Office
- Finance and Grants Management
- Human Resources
- Commercialisation & Affiliations
- Marketing & Communications
- Supporter Services
- Building Redevelopment Project
- Jeans for Genes®
- Community Fundraising
- Philanthropy & Major Partnerships
- Bequests
- Building Foundation Campaign

Management and operations committees

Animal Care & Ethics  Work Health & Safety  Institutional Biosafety  Student admissions  Grants Advisory  Appointments  Promotions

Advisory committees

Scientific Advisory Centre for Kinomics™  Scientific Advisory CellBank Australia™  Cancer Consumer Panel
CMRI is managed by a Board of Directors, consisting of ten independent, non-executive directors and one executive director. Board members bring a wide range of business, commercial and scientific expertise to CMRI.

The role and responsibility of the Board is to identify the critical aspects of organizational governance and to manage the overall business and affairs of CMRI.

Under the Constitution of CMRI, Board members are elected or appointed for three year terms and officer positions are voted on annually. More information on each board member is available online at cmri.org.au/Board and in the Addendum to the 2014 Annual Report available in August.

Directors in Office are:

Professor Frank Martin
(MBBS FRANZCO FRACS AM Oph), President
Professor Martin is currently President of the Asia-Pacific Society of Paediatric Ophthalmology and Strabismus and the International Strabismus Association. He serves on CMRI’s Audit and Risk Committee, Finance and Investment Committee, Intellectual Property Committee, is Chair of the Nominations and Remuneration Committee, and an Executive Member of the CMRI Building Foundation.

Mrs Carolyn Forster
(OAM), Vice President
Mrs Forster worked in the Federal Parliament for 11 years, in the Senate, the House of Representatives, and the Ministry and is a former President of CMRI’s Canberra Committee. She is currently the Australian Delegate to the WFFM. She serves on CMRI’s Finance and Investment Committee, Audit and Risk Committee, and Nominations and Remuneration Committee, and is President of the CMRI Building Foundation.

Mr Rodney Atfield
(FIA FIAA FAIL), Treasurer
Mr Atfield was formerly the Managing Director of the Mercantile Mutual (now ING) group of companies and was Chairman of QBE Mercantile Mutual Limited, as well as a former President of the Institute of Actuaries of Australia and is a Life Member of that Institute. He serves on CMRI’s Audit and Risk Committee, Finance and Investment Committee, Intellectual Property Committee, and the Nominations and Remuneration Committee.

Professor Ian Caterson
(AM MBBS BSc(Med) PhD FRACP)
Professor Caterson is Boden Professor of Human Nutrition and Foundation Director of the Boden Institute of Obesity Nutrition Exercise & Eating Disorders, University of Sydney. He serves on CMRI’s Intellectual Property Committee.

The Hon Craig Knowles
Mr Knowles is the Chair of the Murray-Darling Basin Authority. He was a senior Minister in the New South Wales Government for 10 years, including serving as Minister for Health (1999–2003).

Mrs Patricia Payne
(OAM MPS PhC)
Mrs Payne is currently foundation President of the Women for Pharmacy network and the Australian nominee to the Board of Directors of the Community Pharmacy Section of Federation Internationale Pharmaceutique. She helped form the Hills Committee of CMRI in 1991 and has been its President ever since. She serves as a member of the institutional Biosafety Committee.
Clinical Professor Graeme Stewart
(AM BSc(Med) MBBS PhD FRACP FRCPA)
Professor Stewart was appointed as founding head of Immunology at Westmead Hospital in 1980 and is the Inaugural Director of the Institute for Immunology and Allergy Research, one of the four founding research groups of the Westmead Millennium Institute. He serves as Chair of CMRI’s Intellectual Property Committee.

Professor Roger Reddel
(BSc (Med) MBBS PhD FRACP), CMRI Director
Professor Reddel is a Fellow of the Australian Academy of Science and in 2011 received the NSW Premier’s Award for Outstanding Cancer Researcher of the Year. He heads CMRI’s Cancer Research Unit and has been the CMRI Director and Lorimer Dods Professor, Sydney Medical School, University of Sydney since 2007. He serves on CMRI’s Finance and Investment Committee.

Mr Albert Wong
Mr Wong is Chairman of Winmar Resources Limited and Deputy Chairman of Prima BioMed Limited and Kimberley Diamonds Limited. He has been a Member of the Australian Stock Exchange since 1988 and was the principal of Intersuisse Limited until 1995 when he established the Barton Capital group of companies, including eStar. He serves as an Executive Member of the CMRI Building Foundation.

Mr Bruce Fink
Mr Fink’s career spans 25 years across various fields in the entertainment industry, including cinema and television production. In 2002, Mr Fink established and currently chairs the privately held Bickham Court Group of Companies and is also a co-founder of Media i. He serves on CMRI’s Finance and Investment Committee, Audit and Risk Committee, and is an Executive Member of the CMRI Building Foundation.

Dr Luciano Dalla-Pozza
(MBBS FRACP)
Dr Dalla-Pozza is Department Head and a Senior Staff Specialist of the Oncology Unit at The Children’s Hospital at Westmead. He is an active member of numerous subspecialty societies focused on the care of children and adolescents with cancer.

Mr Michael Loughman
Mr Loughman is the Head of ANZ Private NSW & ACT, which is the Private Banking and Private Wealth Division within ANZ Bank. Mr Loughman is a Banking, Finance and Wealth Management professional with a career spanning 18 years both domestically and globally. He serves on CMRI’s Finance & Investment Committee and the Audit & Risk Committee.

Ms Fiona Crosbie
(BA LLM)
Ms Crosbie is a partner of the international law firm, Allens, where she leads the Competition and Consumer Law practice and serves on the firm’s board. She is also a member of the Competition and Consumer Committee of the Law Council of Australia. She serves on CMRI’s Audit & Risk Committee.
Sprinkled throughout Paul Scully's resume you will see titles like ‘Managing Director’, ‘Chief Executive Officer’, and ‘Non-Executive Director’.

An impressive viewing.

But the position Paul has held as a member of Children's Medical Research Institute's Finance and Investment Committee is the one of most value to us.

He was approached by the committee Chair in 2009 and, familiar with the work of CMRI, he signed on knowing he'd be working toward “worthwhile objectives”.

The committee convenes about half-a-dozen times a year and with responsibility for managing overseas and domestic investments, Paul and other financial minds act on the best interests of CMRI and its future.

“We monitor things like how the portfolio is performing, we look at environmental changes, whether there needs to be investment changes and if the managers appointed are still performing to certain objectives and requirements.”

With no direct plan to tackle the financial world and build his career, Paul’s path into the world of money management was paved around him.

“I never set out with a plan.

“Contrary to what people would do these days in moving from one firm to another, I was with one firm for about 20 years – but it kept reinventing itself.”

And now Paul is witness to and plays a small part in the growth of CMRI.

“I’m happy to be part of CMRI as long as I can make a proper contribution.

“It’s a worthwhile enterprise and I’m glad to be able to help in a small way – it gives me a lot of satisfaction.”

It is a sentiment echoed by co-member of the committee Dr Don Stammer, who was approached, like Paul in 2009, to join the committee.

“I’d been aware of CMRI as one of the favoured charities within medical research in NSW, but I didn’t know all the details.

“The more I learn, the more I’m impressed with the research it’s done and the people involved.”

Don’s resume is as impressive as Paul’s and includes a weekly column in The Australian, weekly broadcasts on the Australian economy and share market for BBC World, and a regular column to the Japanese financial newspaper the Nihon Keizai Shimbun.

His ability to convey complex information to the everyman has helped him enlighten the most scientifically minded on the importance in Australia of franked dividends to zero tax-paying investors such as CMRI.

“I’m a huge supporter of what CMRI is doing and trying to do.

“Working with a group like this, I’m impressed by the quality of research and the quality of people attracted to it.

“To make a limited contribution assisting with investment is very fulfilling.”
Committees & advisory boards

Audit & Risk Committee

The role of the Audit & Risk Committee is to assist the CMRI Board with financial reporting practices and provide advice on operations and risk management strategies. Committee members include:

- Prof. Frank Martin
- Mrs Carolyn Forster
- Mr Rod Atfield (Chair)
- Mr Bruce Fink
- Mr Michael Loughman
- Ms Fiona Crosbie

Nominations & Remuneration Committee

The Nominations & Remuneration Committee assists the Board on Board and Committee appointment practices, succession planning and performance evaluation processes. Committee members include:

- Prof. Frank Martin (Chair)
- Mrs Carolyn Forster
- Mr Rod Atfield
- Mr John Dunlop

Finance & Investment Committee

The Finance and Investment Committee manages and monitors the performance of CMRI’s investment portfolio. Committee members include:

- Prof. Frank Martin
- Mrs Carolyn Forster
- Mr Rod Atfield (Chair)
- Prof. Roger Reddel
- Mr Ralph Mitchell
- Mr Bruce Fink
- Mr Michael Loughman
- Mr Paul Scully
- Dr Don Stammer

IP (Intellectual Property) Committee

The role of the IP Committee is to provide strategic advice on CMRI policy and management of intellectual property. Committee members include:

- Prof. Graeme Stewart (Chair)
- Prof. Ian Caterson
- Prof. Frank Martin
- Mr Rod Atfield
- Mr Ralph Mitchell
Our people

10 of the researchers at CMRI have been here 20 years or more

20 are PhD students newly arrived or here for a few years only

29 are post-docs from a dozen nations, carrying expertise around the world

7 research staff are aged >55; 11 are aged 45-54; 28 are aged 35-44, 29 are aged 25-34, and 15 are aged 18-24

Gender equality

Each year, CMRI submits an annual public report with the Workplace Gender Equality Agency in accordance with the requirements of the Workplace Gender Equality Act 2012 (WGE). CMRI has a long history of encouraging female researchers, and today 56% of our scientists are women. In fact, 63% of all CMRI staff are female.

Managers and senior managers include more women (11) than men (6); however, at the key management personnel and CEO level there is a discrepancy with 5 men and 2 women in these ranks. CMRI will be introducing a gender equality committee in 2015 to find ways to address this discrepancy at senior levels.

Work, Health, and Safety

Children’s Medical Research Institute is committed to ensuring the health, safety and welfare of its workers and visitors. Workers include all staff members, students, contractors and visitors.

A medical research institute like ours has many hazards that need to be managed to keep our workers safe. We have a comprehensive WHS plan and risk management framework that enables us to manage WHS in an efficient and effective manner.

CMRI undertakes the following yearly worker training: General WHS; Emergency and Evacuation; Biological and Chemical Safety; Radiation Safety; and Ergonomics. These topics are covered by a face to face seminar, along with follow up online quizzes. Our emergency wardens also undergo additional training as required by Australian standards.

CMRI has completed many initiatives during the year to support WHS training and management. WHS training seminar attendance is now logged electronically using staff ID badge scanning, allowing easy training record collection and follow up. Initial safety induction training is now completed online using the MindFlash training portal. All new and updated Standard Operating Procedures and Risk Assessments are written using a standardised template, with electronic workflow approval, and are automatically published to ChromoZone, the CMRI intranet, once approved. In the plan for 2015 is a full overhaul of our contractor induction system as well as a full online electronic visitor/contractor check-in system.

CMRI manages consultation on WHS topics through many means. Research and Administration units discuss WHS at their regular meetings. Each unit has a representative that attends a bi-monthly WHS Committee meeting. Other safety related committees where CMRI staff have input are the combined precinct Institutional Biosafety Committee and the CMRI Emergency Planning Committee.

CMRI has a comprehensive online incident reporting system where workers are required to report all safety incidents and near-misses. Management review of incidents and near-misses enable us to continually improve our work environment so it can be as safe as practicable. Bi-annual workplace safety checks are also undertaken with any required remediation carried out as soon as practicable. Numerous staff workplace ergonomic assessments have been carried out with all recommendations being implemented.

CMRI takes pride in the safety culture it harbours. We remind all workers at induction and at our General WHS training:

Mind on Task => Eyes on Task => Safe Outcome
Thank you

We thank the Australian community and our research, business and corporate partners for their ongoing support.

With their help, we can continue to advance the prevention and treatment of disease and create a healthier, brighter future for all children.