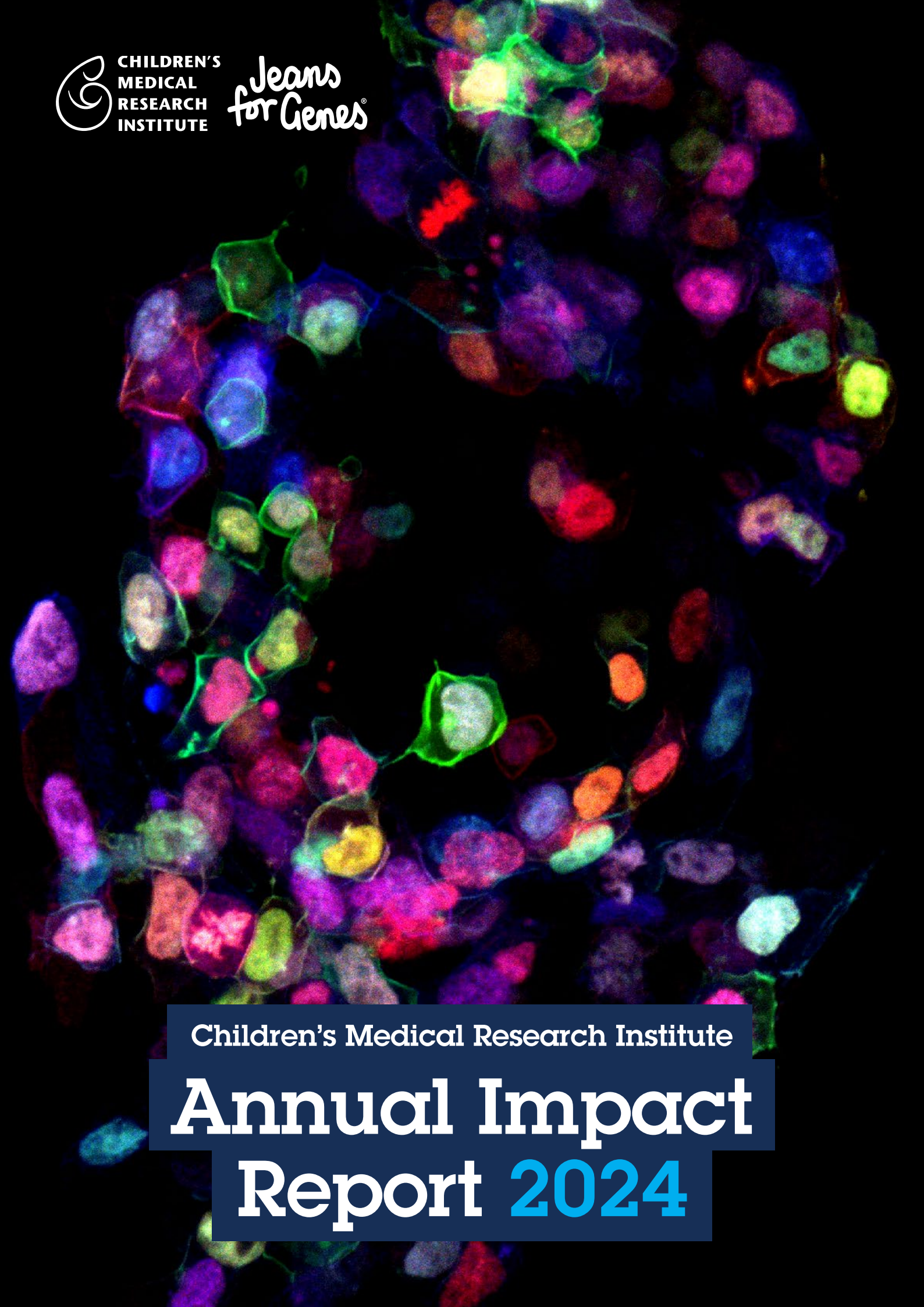
CHILDREN'S MEDICAL RESEARCH INSTITUTE

Jeans for Genes®



Children's Medical Research Institute

Annual Impact Report 2024


Gem, 8
Genetic Blindness

Contents

Cover image: Stem cells visualised under the microscope

From the Director

Welcome to the 2024 Children's Medical Research Institute (CMRI) Impact Report.

Our research programs in our core areas — cancer and genetic disorders/gene technologies — continue to drive major advances globally. These research achievements have been made possible thanks to a mix of competitive grant funding and the ongoing support of our community of donors, trusts and foundations and philanthropists. With Government grant funding increasingly difficult to obtain and funding levels steadily decreasing, the continued support of the Australian public is more than ever crucial to our success.

Our ProCan® cancer research program is making strong progress toward its promise of transforming how cancer is diagnosed and treated. With over 100 national and international collaborations ongoing, we are now preparing to start delivering research results into the cancer clinic. ProCan's progress is described in more detail later in this report. Our telomere and DNA repair research programs have yielded paradigm shifts in our understanding of cancer biology, including finally unravelling the mystery around how cancers respond to radiation treatment that could improve outcomes for very many patients.

We have also had significant wins in the areas of epilepsy research and blinding retinal diseases, as well as contributing to a life-changing discovery that affects how Kabuki syndrome (a serious genetic disease) is treated. Gene therapy research for a range of conditions, such as inherited liver disorders, has had significant boosts, including our world first gene therapy test in whole human liver that was widely reported in the media.

These are just a few of the over 80 discoveries we've published this past year in scientific journals — discoveries that are contributing to the global medical research effort and yielding potential new treatments and cures that will benefit seriously unwell children and their families.

Sincerely,

Professor Roger Reddel AO
Lorimer Dods Professor and Director of CMRI

Introduction

Genetic conditions are devastating for children, their families, carers, and our health system:

1 in 20 children has a birth defect or genetic disease (the leading cause of death in children under 4).

Cancer is mostly caused by genetic changes and is the leading health-related cause of death in **children aged 4–14.**

50% of all admissions to children's hospitals have a genetic contribution, which can affect individuals and their families throughout their life.

Children's Medical Research Institute (CMRI) scientists are working to find treatments and cures to give more of these seriously unwell children a long and healthy life. By focusing our expertise on cancer, genomics and gene therapy, neurobiology, and embryology—we are addressing many of the key areas of need in children's health.

This 2024 Impact Report provides an update on CMRI's overall inputs, activities, and outputs, which lead to our desired impact: achieving our mission of improving the health of children through research.

We employ AAMRI impact metrics (aamri.org.au/members/theresearchimpactproject/), chosen by the medical research sector as suitable indicators of progress towards health impacts.

While this report predominantly uses data and outcomes from the 2024 calendar year, certain metrics, such as citations, necessarily cover a longer period. Impact in medical research takes decades. Long-established research teams at CMRI have had more time to affect health outcomes than have newer teams, but by providing a logic model outlining the pathway to impact, we can still see that even our newest research programs are on track for achieving our desired outcomes.

Jon, Spinal Muscular Atrophy

2024 Impact Highlights

Our People

138
Researchers

34
PhD Students

>700
Collaborations Worldwide

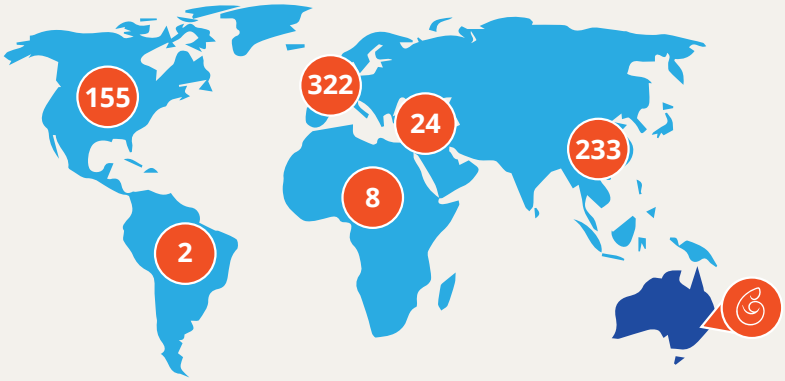
117,000
Supporters in the Australian Community

Research Investment

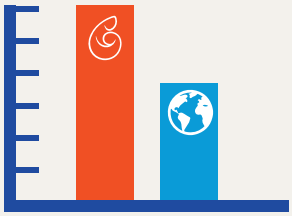
>$6.3 million awarded
Grants (MRFF, NHMRC, ARC)

$38.6 million
Invested by CMRI in Research Activities

Collaborations



Global Impact



Our publication impact (FWCI) is

65% higher

than the global average

57%

of publications in top 10% of journals

>650 million

people worldwide were reached with our news

Commercial Success

TessellateBio

REDEFINING SYNTHETIC LETHALITY

CMRI's spin-out company Tessellate BIO announced a collaboration with Omico, an organisation serving patients with difficult-to-treat cancers in Australia and New Zealand

$238,475

Commercialisation Income

$1,031,676

Sponsored Research

3

New Licences

3

New Patents

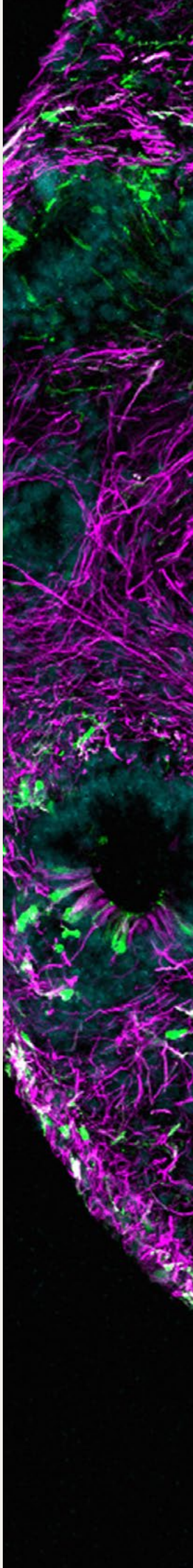
Financials

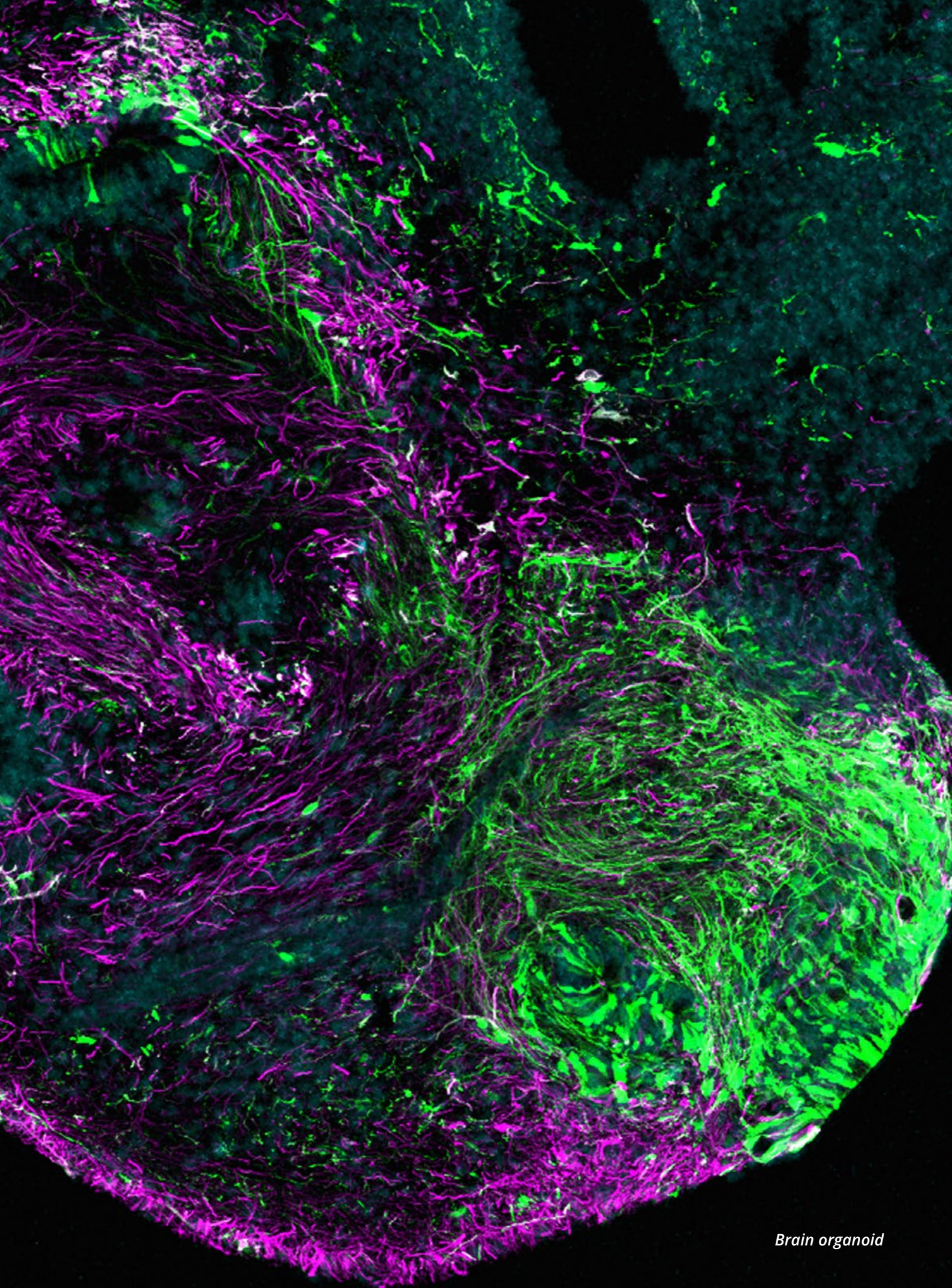
Financial Summary - Profit and Loss Statement

Profit and Loss Statement	AUDITED 31 Dec 2024	AUDITED 31 Dec 2023
	(in $ '000s)	
Revenues		
Research	12,176	25,256
Fundraising	23,861	15,417
Investments and Other Income	4,144	2,871
CMRI Building Redevelopment	6,411	582
Total	**46,592**	**44,126**
Expenses		
Research	38,558	40,612
Fundraising	4,940	4,867
Administration and facilities	4,618	4,434
Total	**48,116**	**49,913**
(Loss)/Income before investment transactions	**(1,524)**	**(5,787)**
Investment transactions, net and impairment	2,938	4,783
Net loss from continuing operations	**1,414**	**(1,004)**
Other comprehensive income	0	0
Total comprehensive loss for the year	**1,414**	**(1,004)**

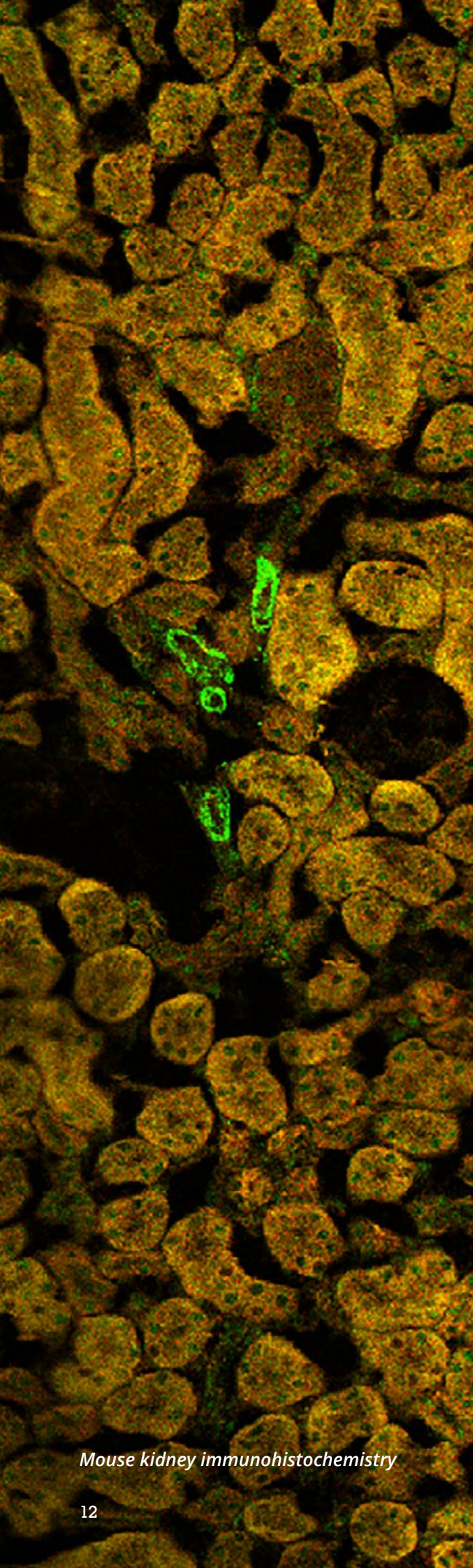
Financial Summary - Balance Sheet

Balance Sheet	AUDITED 31 Dec 2024	AUDITED 31 Dec 2023
	(in $ '000s)	
Assets		
Current Assets, including cash, term deposits & receivables	25,336	25,927
Investment Property	3,178	3,178
Other Financial Assets	73,414	82,780
Property, Plant and Equipment	54,101	48,429
Other Non-current Assets, including receivables	1,883	1,834
Total	**157,912**	**162,148**
Liabilities		
Current Liabilities	13,018	13,143
Non-current liabilities	12,568	18,093
Total	**25,586**	**31,236**
Net Assets	**132,326**	**130,912**



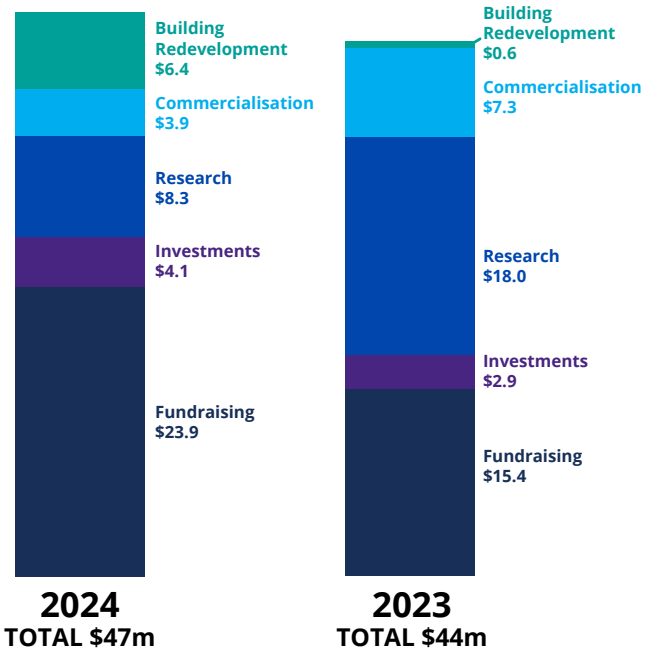


Brain organoid

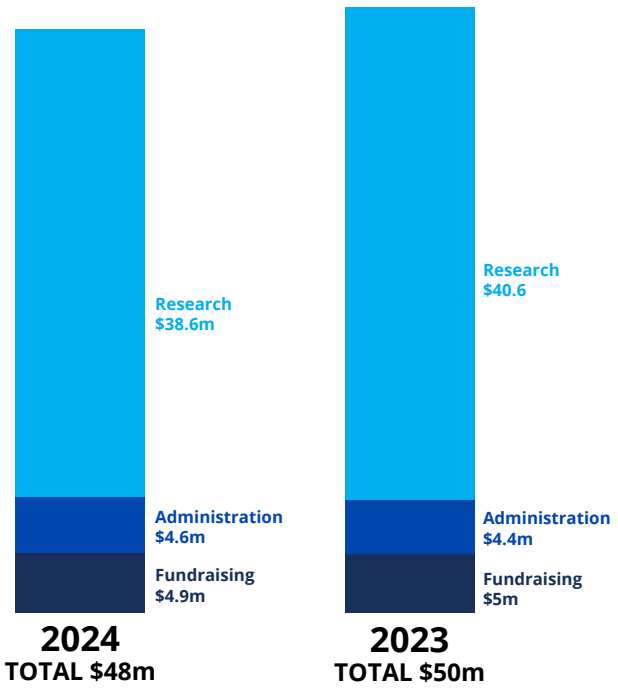


Mouse kidney immunohistochemistry

Sources of Revenue

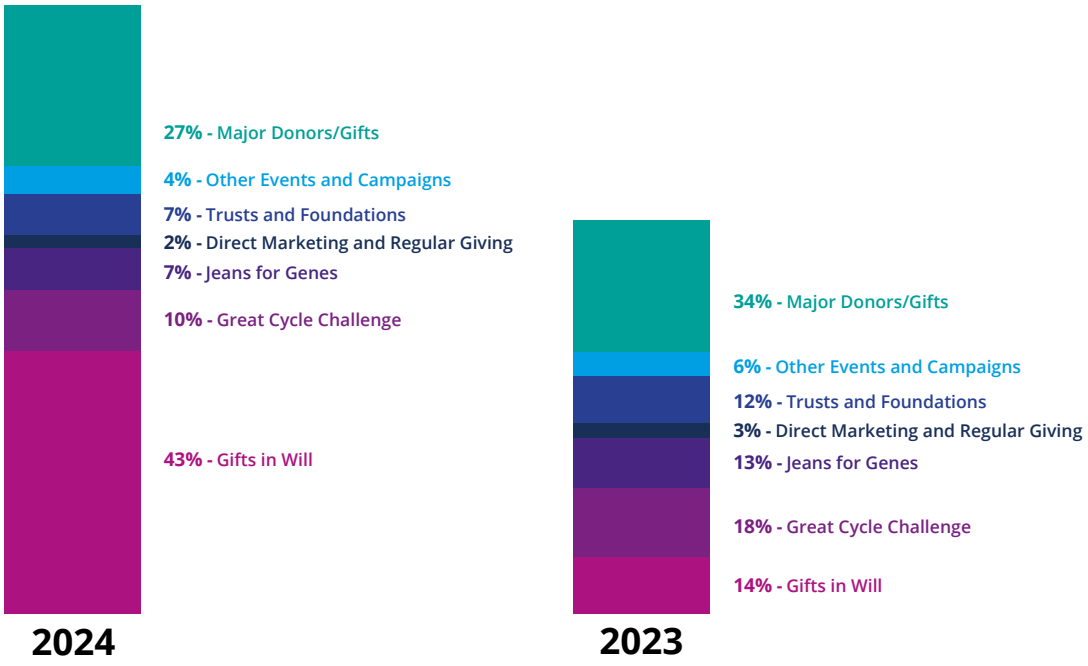


Areas of Expenditure

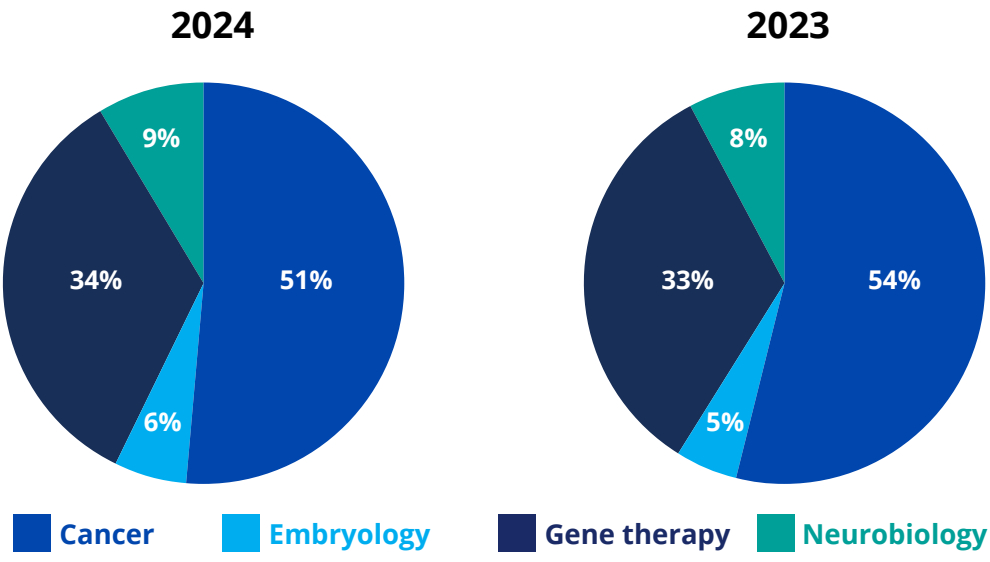


80 cents | from every dollar spent goes to research

Sources of Fundraising Revenue



Research Spend by Disease



Our Impact Model and Metrics for 2024

** all metrics use 2024 calendar year data unless otherwise noted*

Inputs	Participants	Activities

Research Quality

Journals ranked in the

Top 1% 5%

Top 10% 57%

18.8% of our research publications are among the top 10% most cited globally

Field-Weighted Citation Impacts (FWCI): 1.65*

- International collaboration FWCI: **1.92**
- National collaboration FWCI: **1.09**
- Academic-corporate collaboration FWCI: **3.35**

Field-Weighted View Impacts: **1.39****

*65% higher than world average of 1.0
**39% higher than world average of 1.0

Public Reputation

67 years in operation

67% prompted brand awareness for Jeans for Genes

48% prompted brand awareness for CMRI

Financial Inputs

Research Income **$8.3 million** *(includes grants, and Facility sales and services, excluding Commercialisation income)*

Commercialisation Income **$1.9m** *(licences, sponsored research)*

Fundraising Income: **$23.9m**

Investment Income: **$7.1m** *(including revaluation gains)*

Staff

138 Research Staff (60% female)

37 Scientific Support Services Staff (54% female)

75 Non-Research Staff (65% female)

Students

34 Current PhD Students (59% female)

7 PhD Completions

3 Honours Degree Completions

Community Collaborators

Fundraising Committees **14**

Individual supporters **117,000**

Business/School/Organisation Supporters **27,000**

Volunteers **355**

Costs of Research

$38.5 million total

- **$19.8m** Cancer
- **$2.3m** Embryology
- **$13m** Genomics
- **$3.3m** Neurobiology

New Grants Won

Government Grants Totalling $16m won with collaborators (CMRI share $5.8m)

5 Tier 1 Grants:

- **NHMRC $2,177,857** ($681,113 CMRI share, 5% success rate)
- **MRFF $6,532,784** ($945,244 CMRI share, 100% success rate)

NSW Government Grants: $4,997,998 ($1,762,335 CMRI share, 100% success rate)

Trust and Foundation grants: $1,167,293 (25% success rate)

Fundraising Activities

$4.9 million invested

($4.80 raised for every $1 invested)

Communications

>650 million people reached

Indicators quantify or capture each step along our pathway to impact.

The highlights shown below are high-level indicators from 2024. The particular indicators shown here represent a balance between data currently available and the best practice indicators currently being developed by the medical research community, in particular by the Australian Association of Medical Research Institutes (AAMRI) Research Impact Working Group.

Short-term Outputs

80 Publications

Medium-term Outcomes

Commercialisation

Up to 22 November 2024:

$238,475 Commercialisation Income

$1,031,676 Sponsored Research

3 New Granted Patents

3 New Licences/Options

Long-term Impact

Clinically available treatments and cures for children's genetic diseases

Improvements to clinical effectiveness and efficiency

Reduced cost of research or healthcare

New precision cancer diagnostic technologies - to increase cancer cure rates and save more lives

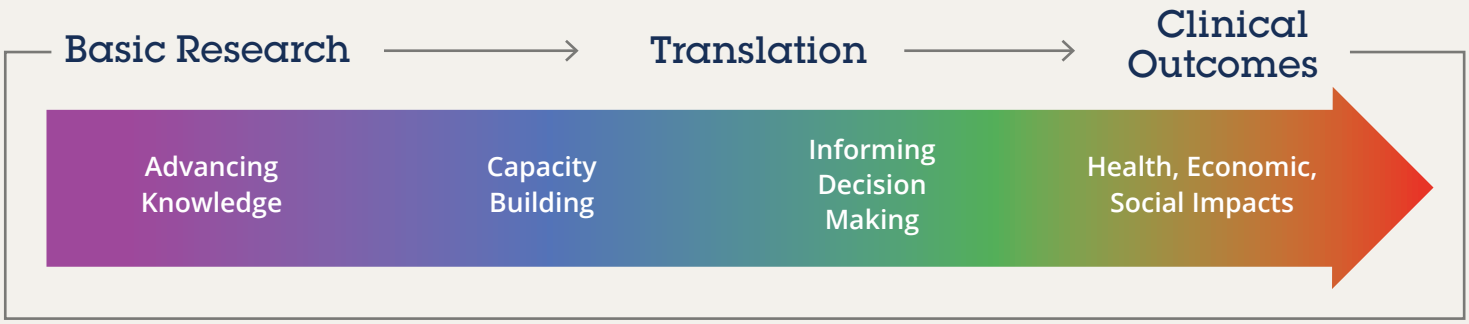
Faster and cheaper stem cell and gene therapy research

New discoveries in fundamental biological processes which can be translated into the clinic

Joseph, Cystic Fibrosis

Key Impacts (bench to bedside) 2023-2024

The below research outcomes have been aligned with the AAMRI Research Impact categories and their position noted on the path from basic research to clinical outcomes.



Developed a new AI based method called Multi-Omic Synthetic Augmentation (MOSA) to address the challenge of incomplete multi-omic datasets using deep learning to generate synthetic data. This innovative approach expanded cancer datasets by 32.7% for over 1,500 cancer cell lines—boosting predictive accuracy and reducing costs.

Federated deep learning model developed to accommodate patient privacy and data security concerns while boosting machine learning capabilities across institutions globally.

Hosted the Sydney International Conference: Advancing Multi-Omics into the Clinic, bringing scientists and clinicians together to discuss Technology, Drug Target Identification, Regulatory Challenges and Commercialisation with discussions and insights that will help shape the future of multi-omics in clinical practice.

Science meets healthcare symposium – collaboration, patient/ family days to engage community.

Advanced fundamental understanding of nerve transmission and how Dyn1xA is crucial for ultrafast endocytosis and brain function, which opens up a range of possibilities for improving epilepsy treatments.

Established multi-year collaboration between Omico and Tessellate BIO (a CMRI spin-out company) which aims to accelerate discovery of synthetic lethal precision therapies to treat cancer.

CMRI's Stem Cell and Organoid Facility became a founding member of NSW Government's new, Non-Animal Technologies Network (NAT-Net) with the goal of reducing the need for animals in research.

Established Australian research priorities for inherited retinal diseases (IRDs) and held a launch at NSW Parliament House. CMRI's A/Prof Anai Gonzalez Cordero was the principal investigator on the project which consulted with patients and families to determine their needs and priorities.

Part of a world first study showing a low carbohydrate, high fat diet had a life-changing impact on children with Kabuki syndrome, using multi-omic sequencing technologies to understand how diet changed gene expression. There are new possibilities for the emerging use of the ketogenic diet and ketones in other epigenetic and neurodevelopmental diseases.

Discovered how different types of radiation therapy can trigger immune responses in cancer and potentially improve treatment outcomes.

Gem, Genetic Blindness

Cancer Collaboration: Basic Science with Clinical Impact


Professor Tony Cesare

Scientists at Children's Medical Research Institute have solved a big mystery in cancer research – why cancer cells die in different ways following radiotherapy. This surprising finding opens up new opportunities to improve treatment and increase cure rates.

The findings were published in *Nature Cell Biology* by first author Dr Radoslaw Szmyd of CMRI's Genome Integrity Unit, which is led by Professor Tony Cesare.

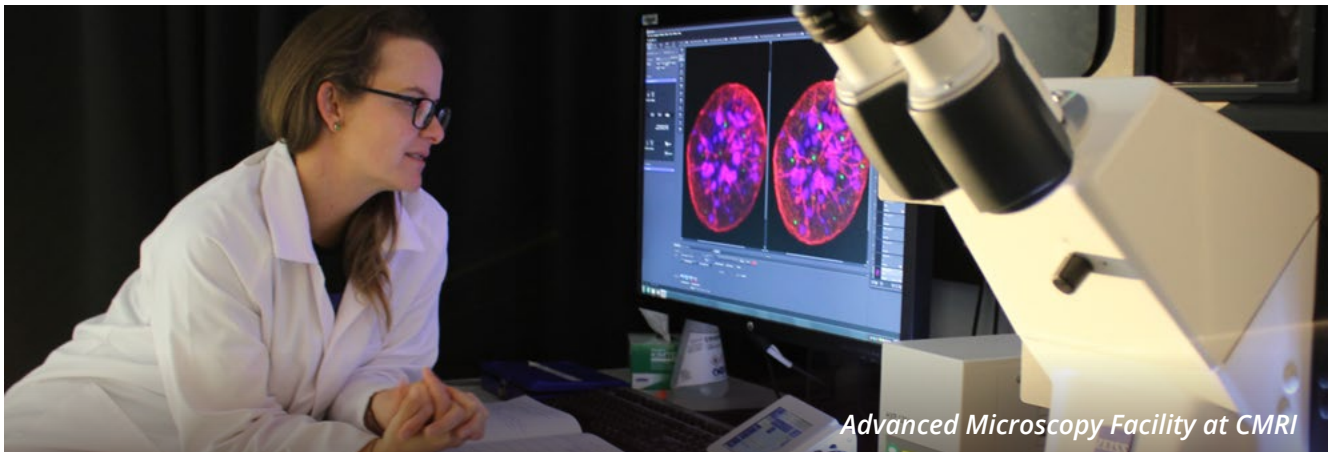
Radiation therapy (also called radiotherapy) is a critically important type of cancer treatment. Scientists have struggled for decades to understand why radiation therapy kills cells from the same tumour in different ways. This is important because some forms of cell death are unnoticed by the immune system, while others trigger an immune response that kills other cancer cells. Unleashing the patient's immune system to kill cancer cells and clear tumours is a major goal of cancer treatment.

"The surprising result of our research is that DNA repair, which normally protects healthy cells, determines how cancer cells die following radiotherapy," said Prof Cesare. "The DNA inside our cells is constantly experiencing damage, and DNA repair is happening all the time to fix that damage and keep our cells healthy. Now, however, it seems these repair processes can recognise when overwhelming damage has occurred (e.g., from radiotherapy), and instruct a cancer cell how to die.

"When DNA damaged by radiation therapy was repaired by a method called homologous recombination (one method of DNA repair), cancer cells died during the process of reproducing—a process called cell division or mitosis. Critically, death during cell division goes unnoticed by the immune system, so it won't activate an immune response. This is not what we want.

"However, cells that dealt with the radiation-damaged DNA through other DNA repair methods survived the cell division process but did so by releasing byproducts of DNA repair into the cell. To the cell, these repair byproducts look like a viral or bacterial infection. This causes the cancer cell to die in a manner that alerts the immune system, which is what we do want."

The team showed that blocking homologous recombination changed the way the cancer cells died—i.e, they now died in a manner that evoked a strong immune response. The team also found that cancer cells that have mutations in BRCA2 – a gene that is very important for breast cancer and which is necessary for homologous recombination – do not die in mitosis following radiotherapy.


Advanced Microscopy Facility at CMRI

Professor Tony Cesare showing cancer cells to a young visitor

In addition to solving a major scientific puzzle, these discoveries will make it possible to use drugs that block homologous recombination to force cancer cells treated with radiotherapy to die in a manner that alerts the immune system to the existence of a cancer (which the immune system had not previously noticed), signalling that the cancer needs to be destroyed.

Prof Cesare credits these breakthroughs to live cell microscope technology that enabled his team to follow irradiated cells for a week following radiation therapy. “Live imaging showed us the full complexity of outcomes following radiation therapy, allowing us to tease out exactly why this occurred.”

Co-project lead, A/Prof Harriet Gee, a radiation oncologist from the Western Sydney Local Health District Radiation Oncology Network, said these findings answer a clinical question that has puzzled the field for 30 years.

“We found that the manner in which tumour cells die after radiotherapy depends on the engagement of specific DNA repair pathways, particularly when radiation is given at very high, focussed doses," said A/Prof Gee. "This opens up new opportunities to enhance radiation efficacy through combination with other therapies, particularly immunotherapy, to increase cancer cures.”

Prof Cesare said Dr Szmyd worked for six years on this “incredibly difficult nut to crack”. Dr Cesare said, “The perseverance required for a project of this scope is a testament to Radek and the team. Everyone is aware of patients battling cancer. Discovering something like this that has the potential to make a big difference to people’s lives is very rewarding.”

Authors on the paper include CMRI researchers Sienna Casolin, Lucy French, Dr Anna Gonzalez-Manjon, Dr Melanie Walter, Lea Cavalli, Scott Page, Prof Hilda Pickett, Dr Chrisopher Nelson, and Dr Andrew Dhawan from the Neurological Institute at the Cleveland Clinic in the US and A/Prof Eric Hau from the Westmead Clinical School at the University of Sydney.

ProCan's Future

The ProCan® program has successfully completed its first phase, having developed a world first, large-scale single-platform approach to obtaining and analysing protein data from cancer samples. What's more, we've developed new methods to understand this massive amount of data that can better diagnose cancers and predict outcomes. The ultimate aim is to enable more personalised cancer diagnosis and treatment planning for every individual patient's cancer.

However, our immediate priority is securing the funding needed to continue the next phase of this ambitious and globally unique program. We need to upgrade our instruments and automate processes, continue to grow the cancer protein database, and develop new algorithms to help clinicians in cancer management.

To this end, we are making some of ProCan's technologies available on a commercial basis to help pay for ProCan's ongoing research. This will leverage ProCan's expertise to help researchers make new discoveries and enable clinical research organisations to achieve greater cancer clinical trial success by better understanding what molecular changes to look for in cancers and which patients are most likely to respond to new therapies being tested. We expect that this will generate revenue to help fund the continuing work needed to build ProCan's database and achieve its core purpose of transforming cancer outcomes for children and adults worldwide.

Growing ProCan's academic and industry partnerships will enable us to keep building our cancer database and develop our customised software into tools for everyday use by cancer clinicians. As ProCan embarks on a new era to develop and implement technologies of direct benefit to patients, we propose to continue the crucial, fundamental research that will answer additional clinically relevant questions and provide the opportunity to make new discoveries. The data we have generated can be mined to reveal new cancer drug targets to study and develop new cancer therapies.

ProCan will continue its trajectory of adding a novel body of knowledge that facilitates other research in Australia and overseas on many diseases beyond cancer and will work with industry partners towards research translation. There will also be opportunities to work with organisations that have already developed new cancer drugs, which are ready for testing: ProCan can speed up the process, making drug development cheaper and faster, so that patients benefit sooner.

The ProCan team recently published a groundbreaking study that uses machine learning to improve proteomic research and identify cancer subtypes. The study introduces ProCanFDL, a Federated Deep Learning approach designed to break barriers in data sharing and facilitates large-scale, multi-cohort analyses while preserving data privacy for proteomics and multi-omic studies.

ProCan scientists are also part of an ambitious new program led by an international consortium, mobilising over 100 cutting-edge experts from academia, government and healthcare industry sectors, which aims to use a combination of proteomics and AI to usher in a new era of medicine and intelligent healthcare.

Called π-HuB (Proteomic Navigator of the Human Body), as outlined in the prestigious journal, *Nature*, the program has several goals including developing a "Meta Homo Sapiens model" which will be a 3D digital representation of human organs, tissues, body fluids and cells over time. This will enable prediction of complex diseases and impacts of non-genetic factors on health.

Recent ProCan Achievements

- Achieved a cumulative total of over 28,000 samples processed
- Over 96,000 sample runs
- 102 collaboration agreements across Australia and 13 other countries with an additional 20 under development
- Total of 156 studies/research projects completed

Harrison, Cancer

Non-Animal Technologies


A/Prof Anai Gonzalez Cordero

Children's Medical Research Institute has a key role in the NSW Government's newly formed Non-Animal Technologies Network (NAT-Net). Our Stem Cell Medicine Group, led by Associate Professor Anai Gonzalez Cordero, is one of the eight founding partners of the Network along with the University of NSW, University of Sydney, University of Technology Sydney, Victor Chang Cardiac Heart Institute, Hunter Medical Research Institute, University of Newcastle and University of Wollongong.

The aim of the new NAT-Net is to develop networking and resources, such as robust cell and computational technologies, that will help medical researchers reduce the need for animals when testing therapies. In addition to NAT-Net, our Stem Cell and Organoid Facility is a crucial contributor to the NSW Organoid Innovation Centre.

Organoids are also known as "laboratory-generated mini-organs". Small samples of skin or blood from patients or healthy individuals are induced in the laboratory to "turn back" into stem cells also known as induced pluripotent stem cells (iPSCs), which can then be directed to become almost any type of cell (nerve, eye, inner ear, lung, kidney cells, etc.), and to then form mini-organ-like complexes, known as organoids.

Organoids offer a unique way to understand human biology, provide unlimited quantities of advanced model systems for research, and are a significant step toward increased use of non-animal models in medical research.

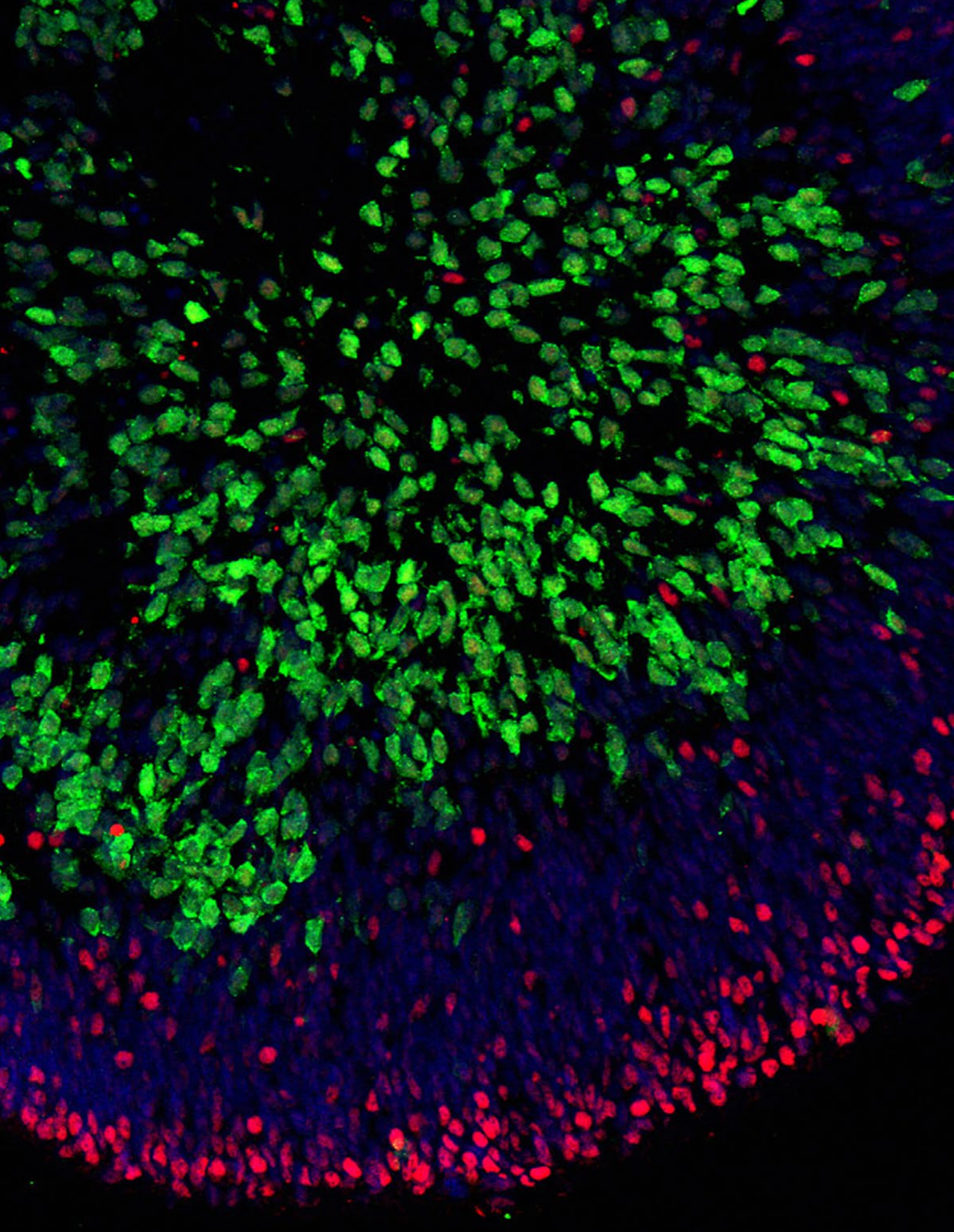
A/Prof Gonzalez Cordero is a leader in the field of stem cells and organoids and said it was a privilege to be a founding member of NAT-Net. Organoids allow researchers to test therapies in a system that is much closer to the human body than animal models. Her speciality area is the human eye and inner ear.

"Lots of therapies fail in clinical trials because the testing is not optimal," she said. "We are working to enhance organoids so that they offer better predictive models for disease.

"It is very rewarding that the NSW Government is recognising the importance of developing these models—this is just the beginning of a very exciting time. It's important for me to emphasise that this initiative will connect researchers across Australia and internationally."

CMRI is a signatory to the Animal Openness Agreement, which means we are transparent in communicating our commitment to the 3 Rs—Reduction, Replacement, and Refinement—of methods requiring the use of animals in research.

Being on the leading edge of non-animal technologies in Australia means CMRI is working on 'Replacement' wherever possible. Yet, there are some research questions that can only be addressed in animals for the foreseeable future—and for ethical and safety reasons there is still pre-clinical research that CMRI conducts in mice and rats to ensure discoveries are safe and effective when they reach clinical trials with children. This means we are equally active in 'Refinement' of our processes to ensure animals are not used unnecessarily, and overall, researchers across Australia have significantly 'Reduced' the use of animals in research by over 50% in the last decade.



Retinal organoid generated from human induced pluripotent stem cells

Amity, CM-AVM2

Alignment with UN Sustainability Goals

CMRI recognises the value of aligning our impact with the UN's Sustainable Development Goals. Sustainable Development Goals (SDGs) provide the world with a "shared blueprint for peace and prosperity for people and the planet, now and into the future." By interpreting CMRI's impact in relation to the SDGs' expert-led framework, we are ensuring that our resources and efforts are working towards lasting, long-term, global systemic change.

CMRI is currently planning and assessing how our priorities align with UN SDGs. A summary of this alignment is presented here. In framing this alignment, CMRI has followed best practice guides such as the UN Global Compact's *Integrating the SDGs into Corporate Reporting* and the Rockefeller Philanthropy Advisors' *Practical Tools for Alignment*. CMRI hopes in the near future to incorporate this framework into future reporting and decision-making.

sdgs.un.org

rockpa.org/project/sdg

CMRI Priorities	Sustainable Development Goals	
Translating fundamental research into health outcomes that improve diagnosis, prevention and treatment of childhood diseases.		Good Health And Well-Being: Ensure healthy lives and promote well-being for all ages.
As a University of Sydney affiliate in the Westmead Research Hub, we are creating a reputation as a global centre of excellence in integrated education, research and healthcare.		Ensure inclusive and equitable quality education and promote lifelong learning opportunities for all.
Improving the health of all children requires enabling the brightest minds, including women and girls, so that they are free to innovate, succeed, and lead.		
Promote translation of discoveries into the clinic for the benefit of patients through commercialisation activities that generate innovative, cutting-edge work with the highest ethical standards of equality and fairness for all.		
Expanding our capacity to provide external researchers with high-tech medical research tools and services through CellBank Australia, the Stem Cell and Organoid Facility, the Vector and Genome Engineering Facility and others.		Build resilient infrastructure, promote inclusive and sustainable industrialisation and foster innovation.
As we expand our building to speed up research, ensure we do so sustainably and for the benefit of future generations.		
Help to protect future generations of children through sustainable consumption.		
Strengthening connections with our many national and international scientific collaborators, the University of Sydney, Westmead Hub Partners, and our paediatric research partners, including the Luminesce Alliance.		Strengthen the means of implementation and revitalise the Global Partnership for Sustainable Development Finance.

Relevant SDG Targets	CMRI Indicators
3.2 End preventable deaths of newborns and children. 3.4: Reduce premature mortality from non-communicable diseases by one third.	# of medicines or treatments where CMRI's role was essential. # of treatments where CMRI's role was essential.
4.4 Increase the youth and adults who have relevant skills.	# of current and graduated students. # recent graduates employed. # instances of staff advancement and upskilling.
5.1 End all forms of discrimination against all women and girls everywhere. 5.5 Ensure women's full and effective participation and equal opportunities for leadership at all levels of decision-making.	% of women at all levels in CMRI % of women using technology to aid remote work and productivity Ensure equal access to conferences and other opportunities for women
8.2 Achieve higher levels of economic productivity through diversification, technological upgrading and innovation. 8.3 Promote development-oriented policies that support productive activities, decent job creation, entrepreneurship, creativity and innovation. 8.5 By 2030, achieve full and productive employment and decent work for all women and men, including for young people and persons with disabilities, and equal pay for work of equal value. 8.7 Take immediate and effective measures to eradicate forced labour, end modern slavery and human trafficking. 8.8 Protect labour rights and promote safe and secure working environments for all workers.	# new jobs created. # spin-off companies created. # translation/commercialisation projects supported by CMRI. Ensure all suppliers used provide equitable and secure working environments for their employees.
9.5 Upgrade the technological capabilities of industrial sectors related to scientific research. Also increasing the number of research and development workers per 1 mil people.	Various indicators relating to CMRI's contribution to the advanced viral vector manufacturing facility. # Research staff and graduates.
11.7 By 2030, provide universal access to safe, inclusive and accessible, green and public spaces, in particular for women and children, older persons and persons with disabilities. 11.A Support positive economic, social and environmental links between urban, peri-urban and rural areas by strengthening national and regional development planning.	Ensure new CMRI building provides green spaces and equitable access to all ages, genders and abilities Work with planning authorities to ensure adequate availability and use of public transportation to connect urban and regional areas
12.5 Substantially reduce waste generation through prevention, reduction, recycling and reuse. 12.6 Adopt sustainable practices and to integrate sustainability information into their reporting cycle and regional development planning.	Ensure new CMRI building meets 4 Green Star rating or higher Support CMRI Sustainability Committee to optimise recycling initiatives as well as review and report on sustainability practices
17.17 Promote effective partnerships, emphasising data, monitoring and accountability.	Commercial and academic collaboration. Involvement with Australian Association of Medical Research Institutes impact indicators working group.

Research Teams

The **Cancer Research Unit**, headed by Professor Roger Reddel AO, conducts trailblazing work on telomeres, which are important for senescence (aging) and all cancers.

Research in the **Cell Biology Unit**, headed by Professor Tracy Bryan, focuses on one of the major factors in at least 85% of all cancers affecting children and adults: the telomerase enzyme.

The **Cell Signalling Unit**, headed by Professor Phil Robinson, studies the detailed molecular mechanisms of how signals are sent from one cell to another in the body and how this impacts a range of diseases.

The **Computational Systems Biology Group**, led by Associate Professor Pengyi Yang, focuses on developing computational and statistical models to reconstruct molecular networks and model their regulations in various cell systems and diseases.

The **Embryology Research Unit**, headed by Professor Patrick Tam, 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.

The **Eye Genetics Research Unit**, headed by Professor Robyn Jamieson, aims to understand the genetic causes contributing to blinding eye diseases to improve the diagnosis and treatment of these conditions.

The **Gene Therapy Research Unit**, headed by Professor Ian Alexander, finds ways to correct genetic diseases in children. The program is a joint initiative of Children's Medical Research Institute and The Children's Hospital at Westmead (Sydney Children's Hospitals Network).

The **Genome Integrity Unit**, headed by Professor Tony Cesare, investigates how cells maintain their DNA health with specific interests in cancer and early development.

There are five **ProCan**® teams working together to analyse tens of thousands of samples representing all types of cancer from all over the world to develop a library of information to advance scientific discovery and enhance clinical treatment worldwide: **ProCan Oncology, ProCan Laboratory, ProCan Cancer Data Science, ProCan Software Engineering and ProCan Operations.**

The **Stem Cell Medicine Group**, led by Associate Professor Anai Gonzalez Cordero, focuses on two complementary fields of research: stem cells and regenerative medicine. Their aim is to disseminate and increase translational stem cell research and utilise the great potential of regenerative medicine for childhood disease.

The **Telomere Length Regulation Unit**, headed by Professor Hilda Pickett, focuses on understanding the molecular mechanisms underlying telomere length regulation and how telomere length can be manipulated to control cell division.

The **Translational Vectorology Unit**, headed by Associate Professor Leszek Lisowski, houses a team of "translational tool makers" who develop and improve vector-based tools that can be used in a wide range of basic and preclinical studies, as well as in clinical applications such as gene therapy.

The **Neuromuscular Gene Discovery Group**, led by CMRI Adjunct Research Scientist Professor Sandra Cooper, is part of the Kids Neuroscience Centre at the Sydney Children's Hospital Network (Westmead), and comprises a large multidisciplinary team devoted to identifying the causes, consequences, and therapies for patients with inherited neuromuscular disorders.

The **Molecular Neurobiology Lab,** led by CMRI Adjunct Research Scientist Associate Professor Wendy Gold, is based at the Sydney Children's Hospital Network (Westmead), and has been studying the pathogenic mechanisms of Rett syndrome for the past nine years.

The newly established **Nuclear Dynamics Group**, headed by Dr Noa Lamm-Shalem, studies the nuclei of cancer cells to understand how they can evade current therapies. They are working to find vulnerabilities in cancers, which could lead to potential new treatments. The team conducts a program of interdisciplinary research spanning fields of neurology, neuropathology, fundamental neuroscience, clinical chemistry, and pharmacology.

Specialised CMRI Research Facilities

Imaging Facility, including the ACRF Telomere Analysis Centre (ATAC), enables world-leading research in the Westmead Research Hub by providing comprehensive support for light microscopy techniques.

Biomedical Proteomics supports projects across CMRI and the Westmead Research Hub, providing equipment, methods and expertise involving MS-based proteomics analysis.

Bioinformatics supports all research at CMRI. It combines information technology with biology, mathematics, statistics and computer science to unravel complex biological problems.

BioResources provides expert animal care services and enforces strict government guidelines to ensure animals are only used appropriately for research purposes.

CellBank Australia™ is Oceania's only registered cell line depository and provides authenticated cell lines and services to the research community, both within Australia and overseas.

The **Single Cell Analytics Facility** provides a range of instrumentation and services for researchers, partner facilities, and commercial entities with the aim to translate basic research into health outcomes that improve diagnosis, prevention, and treatment of childhood diseases.

The **Peptide Synthesis Facility** provides high quality peptide-based materials for researchers, partner facilities, and commercial partners with a primary goal to support cancer research projects across NSW.

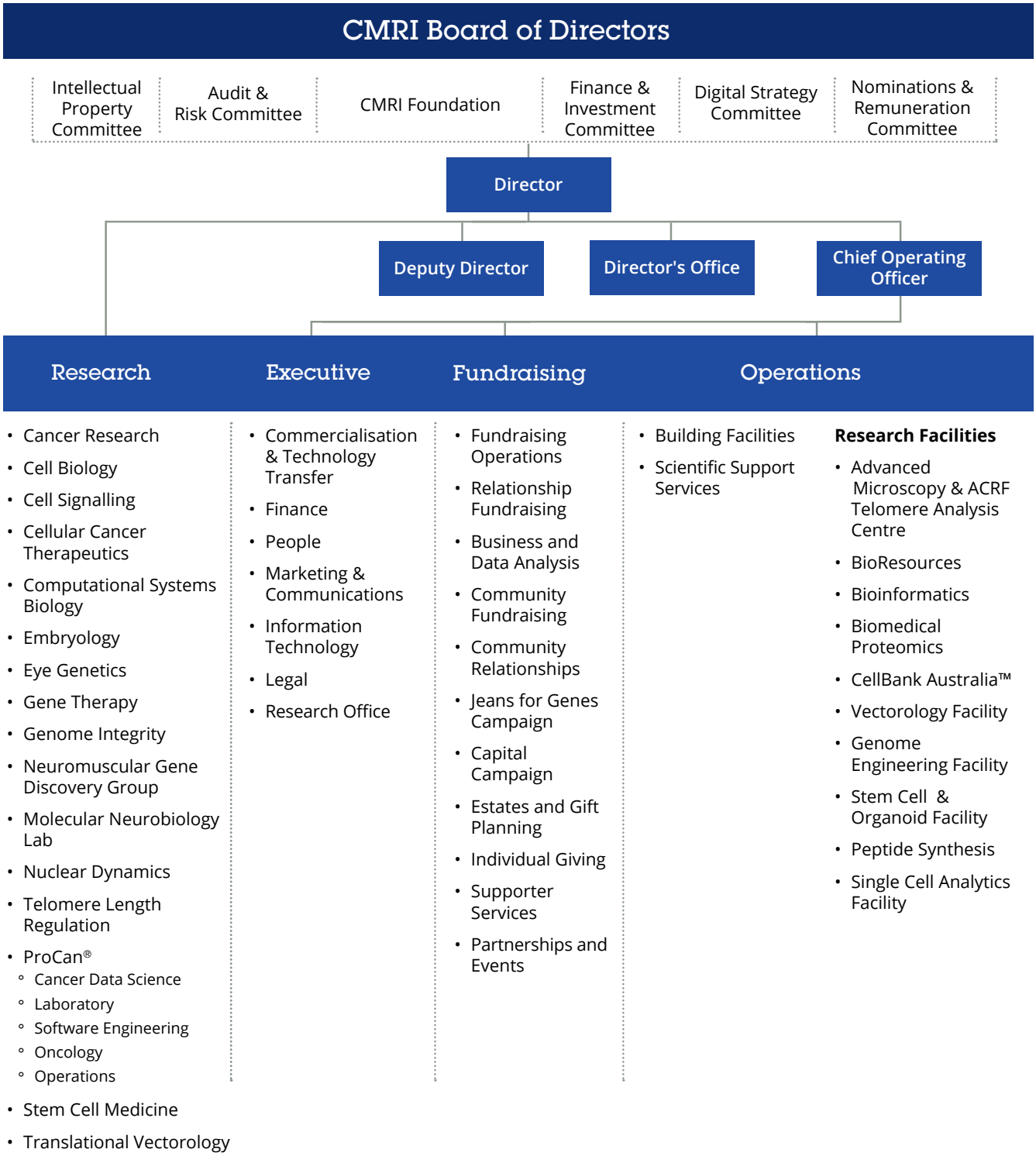
The **Single Cell Analytics Facility** provides a range of instrumentation and services for researchers, partner facilities, and commercial entities with the aim to translate basic research into health outcomes that improve diagnosis, prevention, and treatment of childhood diseases.

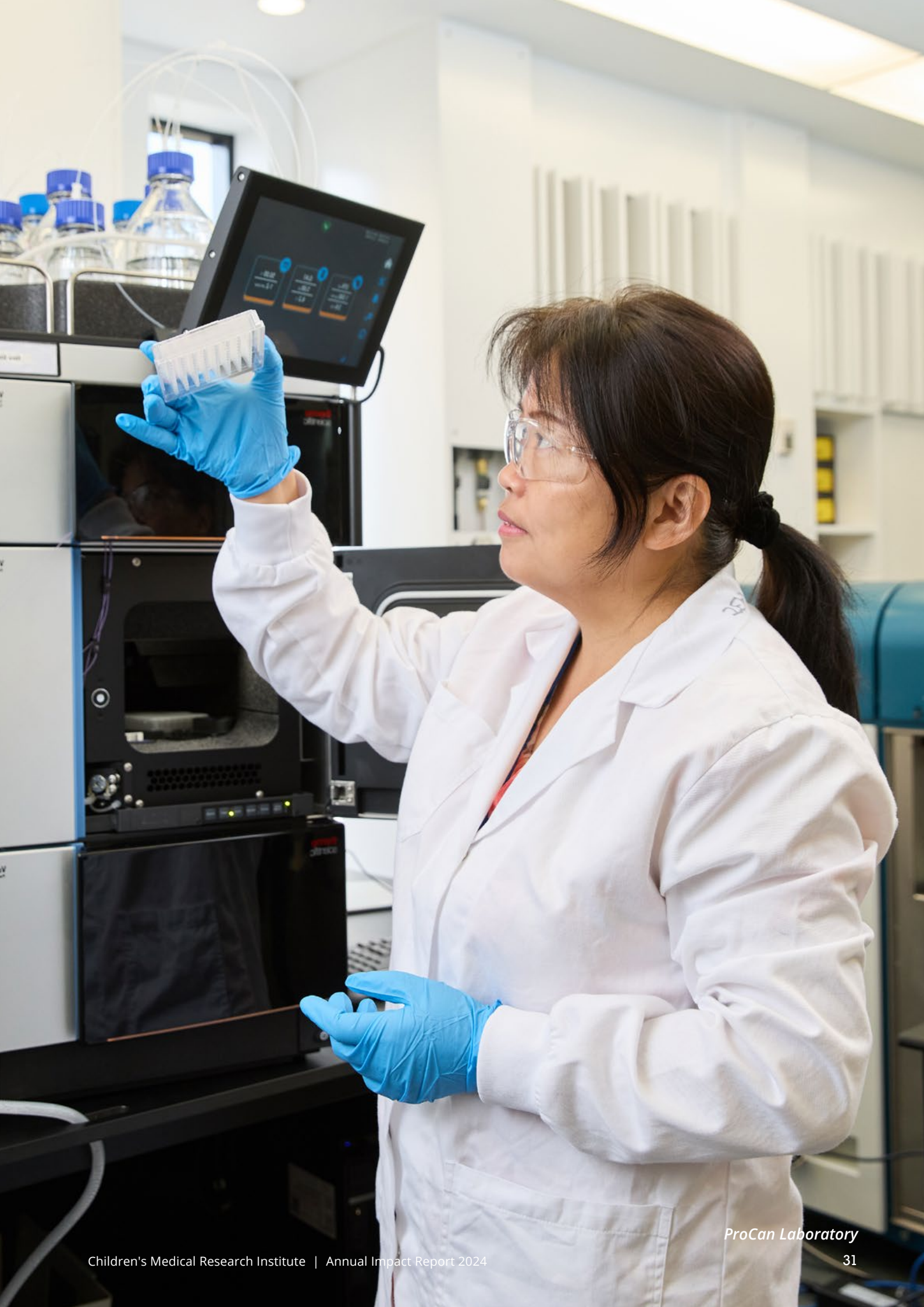
The **Stem Cell and Organoid Facility** offers a range of custom iPSC services including organoid model systems of disease Australia-wide, providing human iPS cells-derived products and derivative tissues to accelerate research Australia-wide.

Vectorology Facility provides commercial services and expertise for AAV or lentiviral vector development, enabling basic and translational research outcomes.

Genome Engineering Facility is proficient in all CRISPR gene editing technologies, providing full service from design to technical support.

Organisational Chart





ProCan Laboratory

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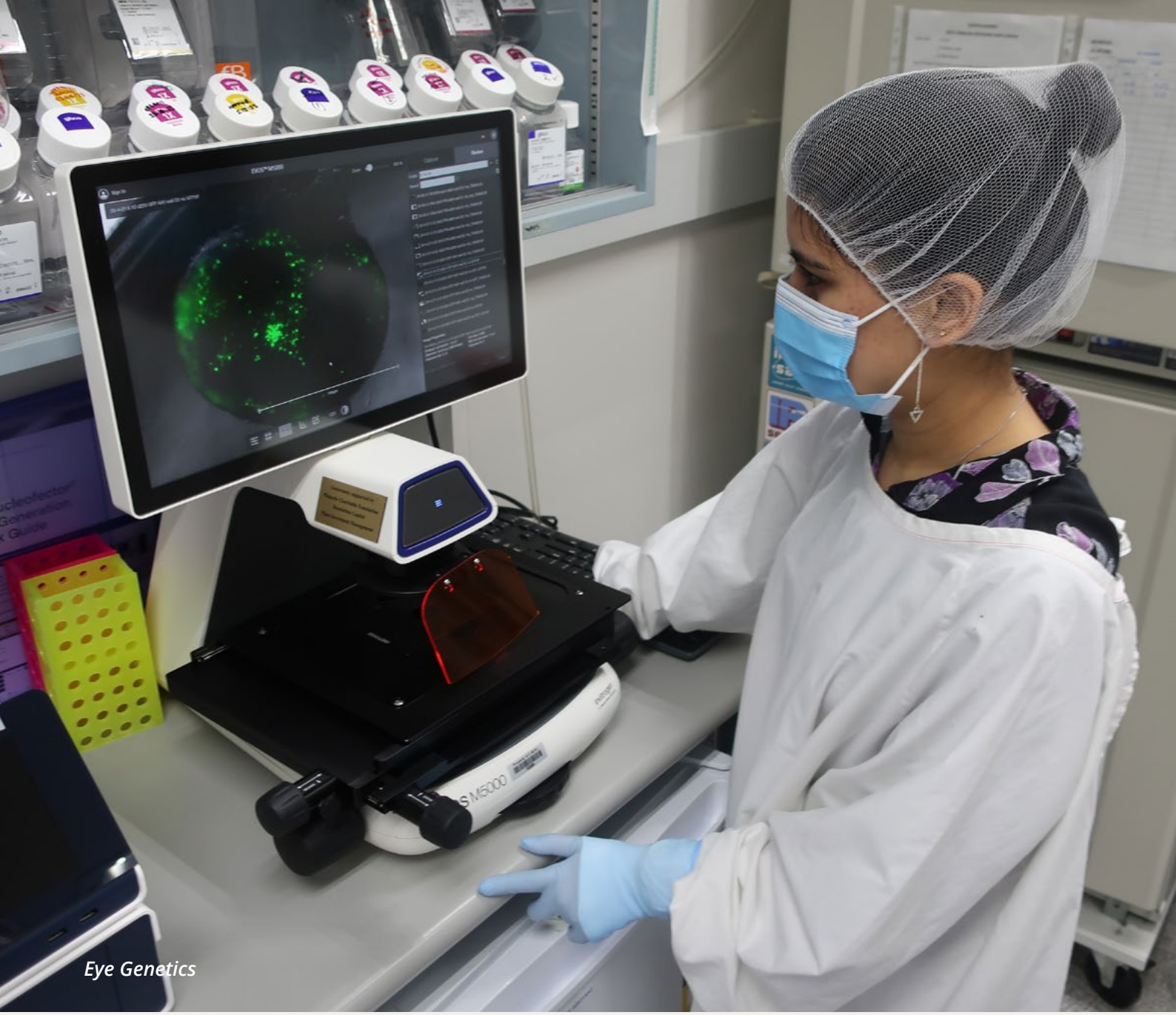

Eye Genetics

Cyber Security

The CMRI Board and its Digital Strategy Committee, together with the Information Technology (IT) team at CMRI have made cybersecurity a priority for CMRI, implementing new systems and processes to ensure all computers and research databases are protected from ransomware or other attacks and that all staff and supporter information remains private. Threats have escalated over the past year, and no organisation is entirely secure, but CMRI regularly monitors and adjusts to ensure we establish and maintain the highest standards of cybersecurity.

Work, Health and Safety

CMRI is committed to ensuring the health, safety and welfare of its workers, contractors, and visitors. CMRI works to safeguard lab worker safety, especially in the areas of Biosafety, Radiation Safety, and Chemical Safety and ensures we are compliant with all institutional, sector, state and national guidelines and laws. CMRI provides health, safety and wellness training, ergonomic safety training and working from home training for all its workers.

Thank you

to our collaborators, supporters, and funders for making our vital work possible.

CHILDREN'S MEDICAL RESEARCH INSTITUTE

Jeans for Genes®

Finding cures for children's genetic diseases

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