Murdoch University

Centre for Molecular Medicine + Innovative Therapeutics

Annual Report 2022

JOINT RESEARCH CENTRE WITH

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EST. 1982 for neurological and translational science

Annual Report 2022

Centre for Molecular Medicine + Innovative Therapeutics

Contents

The 2022 Annual Report differs from that of past years as it is written at a time when leadership of the Centre is changing, with Steve Wilton retiring as CMMIT Director to be replaced by Anthony Akkari in March 2023 (although Steve will continue as Deputy Director). Given these special circumstances, it is appropriate to use the occasion of this Annual Report to acknowledge Steve's contribution as Director as well as include reports from both the Director and Deputy Director, allowing Anthony the opportunity to expand on his vision for CMMIT over the coming years.

Acknowledging our Foundation Director

Professor Steve Wilton was one of the driving forces behind the establishment of CMMIT in 2019 as a vehicle to consolidate Murdoch's research in the field of precision medicine. The other driving force was Professor Norman Palmer, whose contribution cannot be overstated. Over his four years as CMMIT's Foundation Director, Steve oversaw a major expansion in CMMIT's activities, including a 75% increase in the number of research groups and a doubling in the number of postdoctoral scientists. In 2022, CMMIT researchers were involved in grants and industry contracts worth in excess of $25M, making it one of the most successful research centres at Murdoch.

A particular focus of Steve's leadership as Director has been to foster a culture of research translation, particularly commercialisation. Steve was responsible along with Professor Sue Fletcher for the development of three FDA-approved antisense drugs for the treatment of Duchene muscular dystrophy, an achievement that led to both Steve and Sue receiving Orders of Australia (AOs) in 2021.

These successes attracted other researchers in the field of RNA therapeutics so that CMMIT now comprises 8 substantial research groups in the field led by Anthony Akkari, Rakesh Veedu, Sulev Kõks, Steve Wilton, Sue Fletcher and Sarah Rea. The result is a Centre with few parallels in the world. The last several years have seen a steady stream of patents coming out of CMMIT as well as the emergence of multiple spinoff companies in Australia and the USA. Over 50 diseases are currently under investigation as potential targets for antisense therapeutics - so the scene is set for further commercialisation success in the future.

These successes owe much to Steve's leadership. Establishing a new research centre at Murdoch has not been without its challenges, including continuing battles for additional space as CMMIT expanded. Steve's ability to achieve change was greatly aided by the fact that he combined the role of CMMIT Director with that of Director of the Perron Institute, WA's oldest medical research institute. The Perron Institute has been a stalwart supporter of CMMIT from the outset, funding a number of research positions and other initiatives over the years.

Director's Report

Professor Steve Wilton AO
BSc PhD, Director, Centre for Molecular Medicine + Innovative Therapeutics & The Perron Institute for Neurological and Translational Science

It is rather a strange feeling to write my last report as CMMIT Director. The last four years have been something of a blur since CMMIT was first launched by the then Governor of Western Australia, the Hon Kim Beazley in 2019. Over the four years since the launch, CMMIT has performed beyond anyone's expectations as shown in the table across the page.

What sets CMMIT apart from other Murdoch University research centres is that it is a partnership between the Perron Institute - Western Australia's oldest medical research institute – and Murdoch. As a consequence, it draws on the expertise of both organisations and involves researchers and clinicians in a range of diverse fields. The theme that links all of these parts together is personalised medicine, the Centre's ultimate aim being to develop safe and effective treatments tailored to the unique needs of individual patients. The Perron has been a steadfast supporter of CMMIT, and I would like to thank the Perron Institute's Board, its CEO, Steve Arnott and special projects Director, Professor Norman Palmer for their generous support. In addition, words cannot express my gratitude to Jodie Williamson and Bri O'Donnell, CMMIT's Centre Manager and Administrative Officer respectively for their incredibly capable and efficient support in keeping me organised and the Centre running smoothly.

What stands out for me as one of CMMIT's foremost strengths is its connectivity with researchers worldwide. Collaboration in research is important in a variety of ways. It brings together researchers and clinicians from diverse backgrounds with different skill sets to tackle problems from different perspectives. It also creates an environment for the exchange and critique of ideas as so aptly encapsulated in the quote by Francis Crick (opposite page).

An analysis of CMMIT's publications in 2022 shows that the Centre has an exceptional network of collaborative links with institutions around the world, far greater than the majority of research centres. In 2022, it had links with over 90 institutions across Australia and 841 research organisations in 91 countries worldwide. Thus, CMMIT functions as a globally-connected centre in an era when collaboration across borders is often the key to success, all the more important for Australian researchers given our distance from major centres of research in North America and Europe, who still tend to view Australia as 'a galaxy far, far away'.

I hope that CMMIT's focus on research commercialisation will be seen over time as my most important and lasting contribution as Director. There is no other research centre in Western Australia with anything like the same level of commercialisation plus there are few comparable centres across Australia – so CMMIT is justified in claiming to be special. CMMIT's current focus is mainly on commercialisation of antisense oligonucleotides (ASOs) for inherited and acquired diseases. In this area, CMMIT produces multiple patents each year, much of its research is contract research for industry and CMMIT's lead researchers are taking the leap and establishing spinoff companies to commercialise discoveries made in-house at CMMIT. The latest spinoff involving Rakesh Veedu in association with Marvin Caruthers (University of Colorado Boulder) is ProGenis due to be officially launched in 2023.

Whilst CMMIT has been successful in promoting commercialisation, there is always scope for improvement and I am particularly gratified that Anthony Akkari is succeeding me as Director. Anthony's commercial nous built on his years of experience in industry gives him the capacity to take CMMIT to the next level by professionalising the whole commercialisation process, which I am confident will result in CMMIT being an even more formidable force for commercialisation in WA.

Professor Steve Wilton AO

"If, for example, I had some idea, which, as it turned out would, say, be quite wrong, was going off of the tangent, Watson would tell me in no uncertain terms this was nonsense, and vice-versa. If he had some idea I didn't like and I would say so and this would shake his thinking about it and draw him back again. And in fact, it's one of the requirements for collaboration of this sort that you must be perfectly candid, one might almost say rude, to the person you are working with … And this is the end of all real collaboration in science."

Francis Crick

	2019	2020	2021	2022	Increase since 2019
Publications	107	118	157	129	21%
HDR students	30	36	37	40	33%
Research groups	8	10	14	14	75%
Postdoctoral scientists	11	16	21	23	109%
Total grants and contracts	$8.4M	$12.1M	$18.7M	$25.2M	206%
Annualised research income	$2.3M	$5.2M	$16.6M	$8.85M	285%

Deputy Director's Report

Professor Anthony Akkari BSc PhD, Deputy Director, Centre for Molecular Medicine + Innovative Therapeutics

2023 will mark a change in CMMIT's leadership with me taking on the role of CMMIT Director in March when Steve Wilton stands down as CMMIT's Foundation Director.

I must admit that I approach the task of being Director with some degree of trepidation. It is going to be difficult to fill Steve's shoes as he achieved so much in his four short years as Director. During this time, Steve oversaw a major expansion in CMMIT's activities, including a 75% increase in the number of research groups and a doubling in the number of postdoctoral scientists. In 2022, CMMIT researchers were involved in research grants and industry contracts worth in excess of $25M making it one of the most successful research centres at Murdoch.

Establishing a new research centre at Murdoch continues the vision for cutting edge research in health sciences that has without doubt brought significant opportunity and benefit to the university, community and researchers. With this continued success, CMMIT is now focused on maintaining our cutting edge vision in precision medicine and our need to access and grow our laboratory space at Murdoch. Steve's ability to elicit change was greatly aided by the fact that he combined the role of CMMIT Director with that of Director of the Perron Institute and we wish to acknowledge the Perron's generous support over the years.

One opportunity I want to actively pursue as I move into the role of Director, is building on CMMIT's commercialisation capacity in terms of contract research and commercialisable IP. Grants are increasingly hard to get so income diversification must be a priority. Australia's level of research commercialisation is one of the worst in the developed world. Australia's universities are world-class at research but, unlike many universities in Europe and the United States, they are not good at building on this research for commercial returns. My experience in the USA leads me to believe that this can be remedied but this requires researchers to be incentivised and given the support and tools to pursue commercial opportunities. CMMIT's research groups in the area of antisense therapeutics are already paving the way in this regard as evidenced by income from contract research, patents, spin-off companies and commercialisation returns. Other CMMIT groups might benefit over time from a similar approach. I see my role as CMMIT's new Director as continuing this vision of excellence in precision medicine with particular focus on the commercialisation of ASOs and other products of CMMIT's research.

Professor Anthony Akkari

About Professor Anthony Akkari

Many people will know Anthony in his role as Murdoch's Foundation Chair Industrial Pharmacogenetics and Head of CMMIT's Motor Neurone Disease research group, but not everyone will be aware of his background in the pharmaceutical industry in the USA. It is because of the unique set of skills that he gained from his time in industry that he was the obvious candidate to replace Steve Wilton as Director since CMMIT's future lies in focusing more on research translation with a particular emphasis on drug discovery, genomics and commercialisation.

Anthony completed his PhD at the Australian Neuromuscular Research Institute, the predecessor of the Perron Institute. After his PhD, he undertook a post doc position in the department of neurology at Duke University in North Carolina. He then spent over 18 years in industry in the USA where he worked at GlaxoSmithKline, Eli Lilly and Shiraz Pharmaceuticals in the fields of genetics, pharmaco-genetics and personalised medicine. Since moving back to WA, Anthony has established a vibrant research group focusing on motor neurone disease (MND), which has identified and is commercialising a number of compounds with potential for treating MND. He was the driving force behind the establishment of the US-based spinoff company Black Swan Pharmaceuticals where he is Chief Scientific Officer.

Achievements 2022

Grants & Industry Funds

Involved in projects funded from research grants & industry contracts worth:

$25.2M

This represents a 37.4% increase in total research income compared to 2021.
Many grants involved multiple institutions & consequently only a portion of the funds came to CMMIT

CMMIT researchers involved in grants commencing in 2022 worth:

$8.85M

Including; Medical Research Future Fund $4.665M
Channel 7 Telethon $1.047M
NHMRC $1.043M
WA Innovation Fund $0.979M
Multiple Sclerosis Research Australia $0.455M

CMMIT undertook

$2.421M

of contract research for companies and investors in Australia, USA and UK:
Several of these projects were industry-funded clinical trials in the area of advanced wound management

Core funding from CMMIT's joint research centre partners, Murdoch University & the Perron Institute:

$2.647M

Murdoch also provides on-campus facilities & equipment

Publications & Patents

Researchers published:
129 scientific papers
some in high-impact journals such as:
Lancet (IF: 202.7)
BMJ (IF: 96.2)
Nature (IF: 69.5)
Nature Genetics (IF: 39.9)
Lancet Global Health (IF: 38.9)

Continued involvement in:
3 spinoff companies
Including Black Swan Pharmaceuticals, SynGenis & RAGE Biotech
+
a further spinoff, ProGenis Pharmaceuticals to launch in 2023

CMMIT researchers were involved in:
5 new patents
Multiple patents are being actively commercialised

Awards

- **Steve Wilton AO & Sue Fletcher AO - AusBiotech Johnson & Johnson's Industry Leadership Award**
- **Sue Fletcher AO - inducted Fellow of the Australian Academy of Health & Medical Sciences**
- **Kylie Sandy-Hodgetts - Winner World Union of Wound Healing Societies Innovation in Surgical Site Infection**
- **Craig McIntosh - 2022 AGCTS Early Career Researcher Travel Award**
- **Aidan Murphy - 2022 AGCTS Student Poster Travel Award**
- **Marzena Fabis-Pedrini - 2022 Perron Aspire Award Winner**

Positions and Staff breakdown

CMMIT has grown to include more than 60 Academics, Professional & Technical Staff and now supports 40 post-graduate students

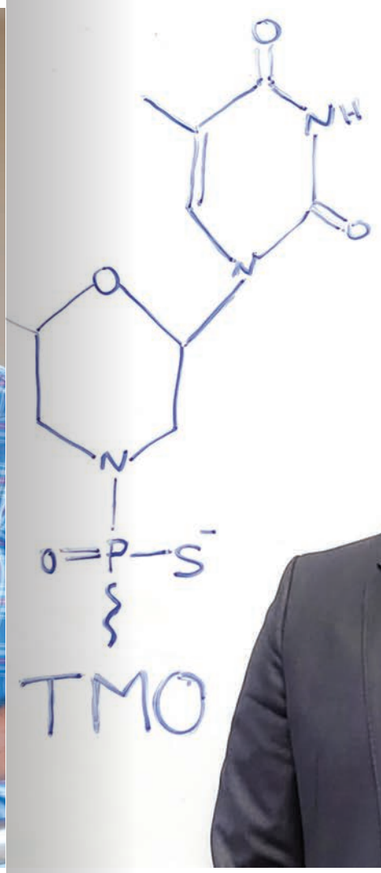
21	23	18	40	60%	40%
Academics/ Group Leaders	Postdoctoral Fellows	Professional & Technical Staff	PhD & Masters by Research Students	Females	Males

Research Highlights

Over the coming pages are selected examples chosen to showcase the diversity, quality and impact of CMMIT's research.

The basic premise that underpins CMMIT's research is the belief that we must first understand the processes involved in disease pathogenesis before we can begin to design targeted therapeutics and diagnostics. Only then can we begin the process of research translation and commercialisation.

Our research on the role of transposable elements in Parkinson's disease, origins of MS, frontotemporal lobar degeneration and amyotrophic lateral sclerosis are all examples of CMMIT's research on disease pathogenesis. Similarly, our research on thiomorpholino ASOs and the early molecular diagnosis of Western Australian children with undiagnosed rare diseases are just two examples out of many of CMMIT's research on therapeutics. At the clinical end of the spectrum, our research on the therapeutic benefits of yoga in veterans and on clinical trials for new approaches to wound management illustrate CMMIT's commitment to clinical translation.



Project Snapshots

FRONTIERS IN WOUND MANAGEMENT

The Skin Integrity Research Group's research program headed by Kylie Sandy-Hodgetts, is multi-centred, multidisciplinary and conducted across national and international healthcare settings. Kylie is chief investigator of several industry-funded clinical trials totalling over $3.2 million, including the EDISON, CRISTINA and CRISP trials, which are investigating the early detection and prevention of wound complications after surgery. Some of these trials have international participating sites and involve HDR students. Kylie is also the Chair of the Australian Health Research Alliance (AHRA) Wound Initiative Surgical Wound Panel Steering Committee, which is developing research priorities for Australian wound care.

Kylie is leading a new educational initiative with key healthcare stakeholders in the UAE and wider region focusing on the launch of a new Murdoch University Masters in Advanced Wound Care, the first of its kind in the region. This initiative is significant for the UAE and adjoining regions as there is growing demand for practitioners in this speciality.

Kylie remains heavily involved in the International Surgical Wound Complications Advisory Panel, a global not-for-profit organisation that aims to raise awareness of the need for early identification and prevention of surgical wound complications.

NEW APPROACH TO PARKINSON'S DISEASE

Transposable elements (TEs) – DNA sequences capable of changing position in a genome - make up over 70% of the human genome and until recently were considered 'junk' DNA without any meaningful function. The advent of genomics has shed new light on the activities of TEs suggesting that their role is far from marginal.

Studies at CMMIT in collaboration with the University of Liverpool are focusing on the role of TEs in Parkinson's disease (PD) using samples from an international patient cohort. This research has identified several TEs that increase the progression of PD with concomitant faster neuronal loss in the brain. At the same time, other TEs have been identified that appear be protective as their presence slows down progression of the disease. New genetic pathways have been identified that determine the trajectory of PD and go some way to explaining individual differences in progression of the disease. Understanding the molecular mechanisms underlying the effects of TEs is helping the group identify the novel genomic or transcriptomic factors behind these differences. This knowledge has helped to identify the genes and transcripts that protect neurons from damage and has been decisive in developing new therapeutic approaches for Parkinson's. The research findings also appear translatable for dementias and other neurodegenerative conditions. This work is being undertaken by the Neurodegenerative Diseases Group led by Professor Sulev Kõks, ably assisted by Dr Abi Pfaff and team.

THE SEARCH FOR THE NEXT GENERATION OF ANTISENSE THERAPEUTIC

Much of CMMIT's research uses antisense oligonucleotides (ASOs) - short, RNA-like molecules that interact with mRNA - to modify the expression of disease-causing genes. ASOs are increasingly being used to treat a range of diseases. One type of ASO in common use is the phosphorodiamidate morpholino oligomer (PMO) but PMOs have significant limitations, particularly poor cellular uptake and high cost.

CMMIT researchers led by Rakesh Veedu and Bao Le in collaboration with colleagues from the University of Colorado have led the search for the next generation of ASOs with improved efficacy, safety, and biodistribution. Their research published in the highly prestigious journal *Proceedings of the National Academy of Science* suggests that a new class of ASO based on thiomorpholino (TMO) chemistry is not only effective at modifying gene expression but is more cost-effective as they are easier to synthesise and require lower doses.

What remains to be established is their activity and toxicity in vivo relative to other chemistries, but preliminary indications are that TMOs may represent the elusive next generation of ASOs.

"The breadth of CMMIT's research leads to good science outcomes and ultimately better healthcare."

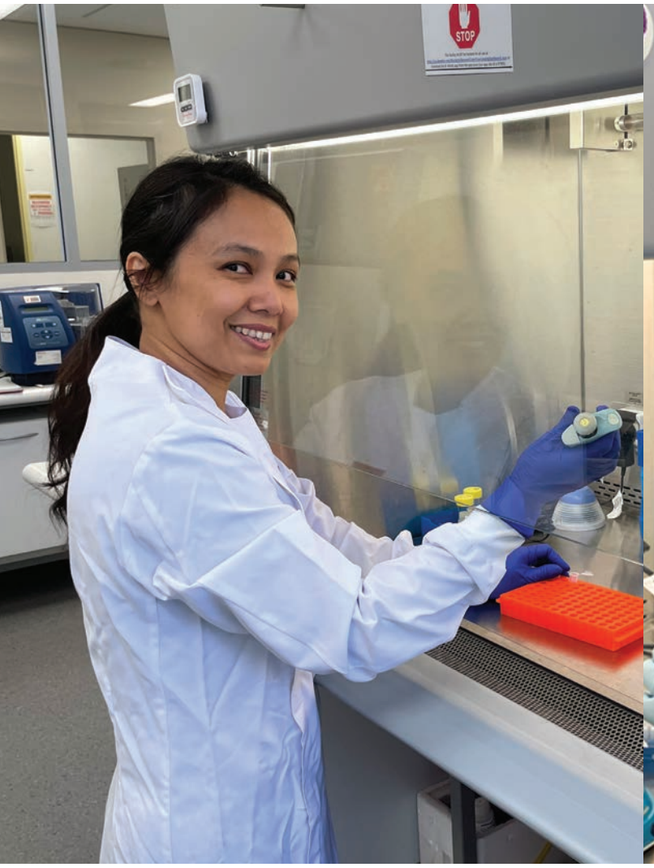


Image courtesy of the Perron Institute

HOPE FOR THOSE WHO HAVE NONE

Discovering ground-breaking new treatments for childhood diseases is the focus of a pioneering project underway in WA, thanks to a major grant from the Channel 7 Telethon Trust. This major funding boost enables researchers to find treatments for rare childhood genetic disorders that currently lack any effective therapy.

With over 63,000 children in WA diagnosed with a rare disease, there is a pressing need to find novel ASO treatments for children with undiagnosed genetic disorders. This is challenging as it currently takes on average 5 years to get a diagnosis for a child with a rare disease, during which time the disease progresses, often irreversibly. The solution is to shorten the time between initial patient consultation, diagnosis and treatment and, this is the focus of the Telethon project. Working with clinicians, CMMIT researchers led by May Aung-Htut and Steve Wilton are focusing on the early molecular diagnosis of children with clinically diagnosed rare diseases to precisely identify the underlying genetic defect so that ASO drug design can begin as early as possible.

After testing in patient cells, ASOs will be evaluated clinically in investigator-initiated clinical trials. The ultimate aim is to shorten the time gap between diagnosis and therapy so that more affected children receive treatment as soon as possible.

FUNCTIONAL GENOMICS: AT THE FOREFRONT OF BENCH TO BEDSIDE RESEARCH TRANSLATION

The Functional Genomics Group aims to understand the mechanisms leading to the development of amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD) and Paget's disease of bone.

In 2022 they reported that specific inherited changes (mutations) in the *TBK1* gene caused distinct functional effects in cells. Some *TBK1* mutations increased neuroinflammation, others reduced the capacity of cells to remove disease-associated proteins (via autophagy). Their results suggest that no single pathway leads to disease in individuals with *TBK1* mutations.

In a separate clinical trial, they examined whether zoledronic acid affects patients with Paget's disease of bone. Zoledronic acid is a bisphosphonate that prevents bone removal. The trial showed that a single dose in individuals with genetic predisposition, prevents disease development. These findings will change clinical recommendations for individuals with a family history of Paget's.

The group secured significant research funding to perform preclinical testing of our best drug candidates in cell and animal models of ALS-FTD, and to develop more effective methods of delivering drugs into the central nervous system.

YOGA FOR MENTAL HEALTH IN VETERANS AND FIRST-RESPONDERS

Dr Danielle Mathersul, a group of current and ex-serving members of the Australian Defence Force and first-responders have worked together to co-create a 10-week yoga program for mental health and resilience. The project received funding from a CMMIT Internal Project Grant and involved collaboration with fellow researchers in CMMIT's Clinical Exercise and Cognition group and other WA universities.

Australian Veterans and first-responders have one of the highest rates of mental health disorders and suicide. The interim report from the ongoing Australian Royal Commission into Defence and Veteran Suicide emphasises the need for significant changes to existing services to help individuals better cope with stress and trauma. Research suggests that yoga may be beneficial. While most USA-based Veteran hospitals offer yoga, it is not funded by the Australian Government or private healthcare services.

With the aim of changing these policies, Danielle and her team are now conducting a trial to evaluate the efficacy of the co-designed yoga program for mental health and resilience.

DECODING MS: INVESTIGATING LYMPHOCYTE TARGETS IN THE BRAIN

Funding from the US-based National Multiple Sclerosis Society is supporting work led by Clinical Professor Allan Kermode and Dr Belinda Kaskow in collaboration with CMMIT researchers Associate Professor Abha Chopra, Dr Xiaonan Zhong and Dr Marzena Fabis-Pedrini.

Their work involves understanding the link between Epstein Barr Virus, which spreads through saliva, causing glandular fever, and Multiple Sclerosis (MS). MS is a condition where lymphocytes (immune cells) attack self-antigens (cellular proteins) in the brain possibly mistaking them for the Epstein Barr-Virus. In this project, they will look at the lymphocytes present in the damaged areas of the brain from donated post-mortem samples of individuals with MS to determine whether these lymphocytes are targeting proteins native to the brain or foreign invaders. Additionally, the team will screen blood samples of individuals recently diagnosed with MS to determine if these lymphocytes can be detected before they go to the central nervous system.

This critical project aims to unravel the causes of MS, leading us closer to developing more effective and innovative treatments.

Training the Next Generation

Image courtesy of the Perron Institute

In 2022, CMMIT had 40 Higher Degree by Research (HDR) students undertaking Masters and PhD degrees.

HDR students – including many from overseas – constitute the largest single group within CMMIT, accounting for over 25% of its total workforce. Training HDR students is one of the key responsibilities of any research organisation and CMMIT is proud of the support it provides to HDR students.

Doing a higher research degree is invariably a challenging personal journey for any student and here we showcase two students, Dr Frankie Theunissen and Dr Di Huang, who completed their PhDs in 2022.

FRANKIE THEUNISSEN - A PERSONAL JOURNEY

Frances (Frankie) Theunissen has just completed her PhD at CMMIT/Perron Institute where her research sought to identify novel genetic markers for motor neurone disease (MND) that can be used as enrichment tools to improve the success of MND clinical trials.

In 2022, she published 2 papers in high impact journals (an invited expert opinion in BMC Medicine and an experimental manuscript in Scientific Reports) and was a finalist in the PhD category for the 2022 Premier Science Awards, judged across all STEM students in WA. In November, she submitted her PhD thesis titled 'Characterisation of novel genetic markers for sporadic amyotrophic lateral sclerosis', which was comprised entirely of publications, including 5 first-author publications, 5 collaborative publications and one international patent. During this time, Dr Theunissen also formed and led international collaborations to launch a genetic marker pipeline project involving funding of $250,000 from FightMND (2022-2024).

As a result of this collaborative project, Dr Theunissen has been invited to undertake postdoctoral training at King's College London where she will work in the world-leading MND genetics laboratory led by Professor Ammar Al-Chalabi.

DI HUANG - FROM A CAREER IN MEDICINE IN CHINA TO ONE IN MEDICAL RESEARCH IN WESTERN AUSTRALIA

Di Huang is a medical graduate from China who completed her medical degree in Sichuan before undertaking a Master of Ophthalmology degree at Kunming Medical University - so her PhD journey caused her to move countries and move away from clinical medicine into the sphere of medical research, although she continued to focus on eye diseases.

Di Huang undertook her PhD in CMMIT's Molecular Therapy supervised by Steve Wilton, Sue Fletcher and May Aung-Htut as well as Fred Chen and Sam McLenachan from the Lions Eye Institute. Her research focused on applying antisense oligonucleotide (ASO) technologies to treat Stargardt disease (STGD1). STGD1 is a rare and progressive genetic eye disease that causes vision loss and eventually blindness. There is no approved treatment. Studies in patient skin cells led to the discovery of an ASO with the potential to halt the progression of vision loss in some patients. During her PhD, Dr Huang published seven papers, six as the first author. She also received multiple prizes in 3-minute Thesis competitions, including the Virtual Asia-Pacific. Dr Huang is currently working with Fred Chen and Sam McLenachan from the Lions Eye Institute on developing a new platform (retinal organoids) built from induced pluripotent stem cells obtained from patients with inherited eye diseases.

Our Networks & Links

Science by its very nature depends on collaboration between scientists from different backgrounds and different institutions.

The simple fact is that the hardest problems in science can only be solved by open worldwide collaboration. Against this background, there is increasing recognition of the importance of collaboration as a way of measuring the impact of an organisation's research. Collaboration can be measured in a variety of ways, but one of the most robust measures is the number of publications involving collaborators from other institutions, particularly from overseas.

By this metric, CMMIT collaborated with:

- **All of WA's universities, research institutes & major hospitals**
- **Nationally with over 91 institutions across all Australia's States and Territories**
- **Internationally with 841 institutions from 81 countries in every continent except Antarctica**

96% of CMMIT's 2022 publications involved co-authors from other institutions with 65% involving co-authors from overseas.

The world map above shows the extent of the geographical spread of CMMIT's collaborative links in 2022.

Another facet of collaboration is the involvement of researchers in international research consortia focusing on specific issues in healthcare. CMMIT's researchers are involved in consortia focusing on Parkinson's disease, multiple sclerosis and myositis as well as a number focusing on health economics and global health issues. 2022 saw the emergence of the COVAD Study Group involving Merrilee Needham, an international consortium examining the impact of COVID-19 vaccination in patients with autoimmune diseases.

Collaboration is important in a variety of ways. It brings together researchers from diverse backgrounds and with different skill sets to tackle a problem from different perspectives. It also creates an environment for the exchange and critique of ideas.

Outreach & Impact

CMMIT believes that medical research cannot take place in an academic vacuum but must involve the community and consumers in all aspects of research. To this end, CMMIT formed a Community Advisory Group some years ago and 2022 saw the rollout of other initiatives aimed at increasing community involvement.

Community Conversation

CMMIT continued its partnership with the Western Australian Health Translation Network's Consumer and Community Involvement Program, facilitating a Community Conversation about how molecular medicine can improve the lives of people with rare diseases.

The Conversation allowed community members who have lived experience or care for someone with a rare disease to hear first-hand from CMMIT researchers who are working hard to ensure no rare disease patient gets left behind, and that safe and effective treatments can be found over time for every patient.

The Conversation was designed for people with rare disease, care-givers and the general community to learn about the personalised treatments CMMIT is developing, what these medicines can and cannot do, and what challenges researchers need to overcome before a 'drug for every child with a rare disease' becomes a reality.

The event struck a chord with community members and highlighted the need for CMMIT to communicate its research so that people are aware of developments.

"The best products are born from a deep empathy with the people who use them."

Bill Buxton,

Partner Researcher at Microsoft

Outreach & Impact

Tours and visits

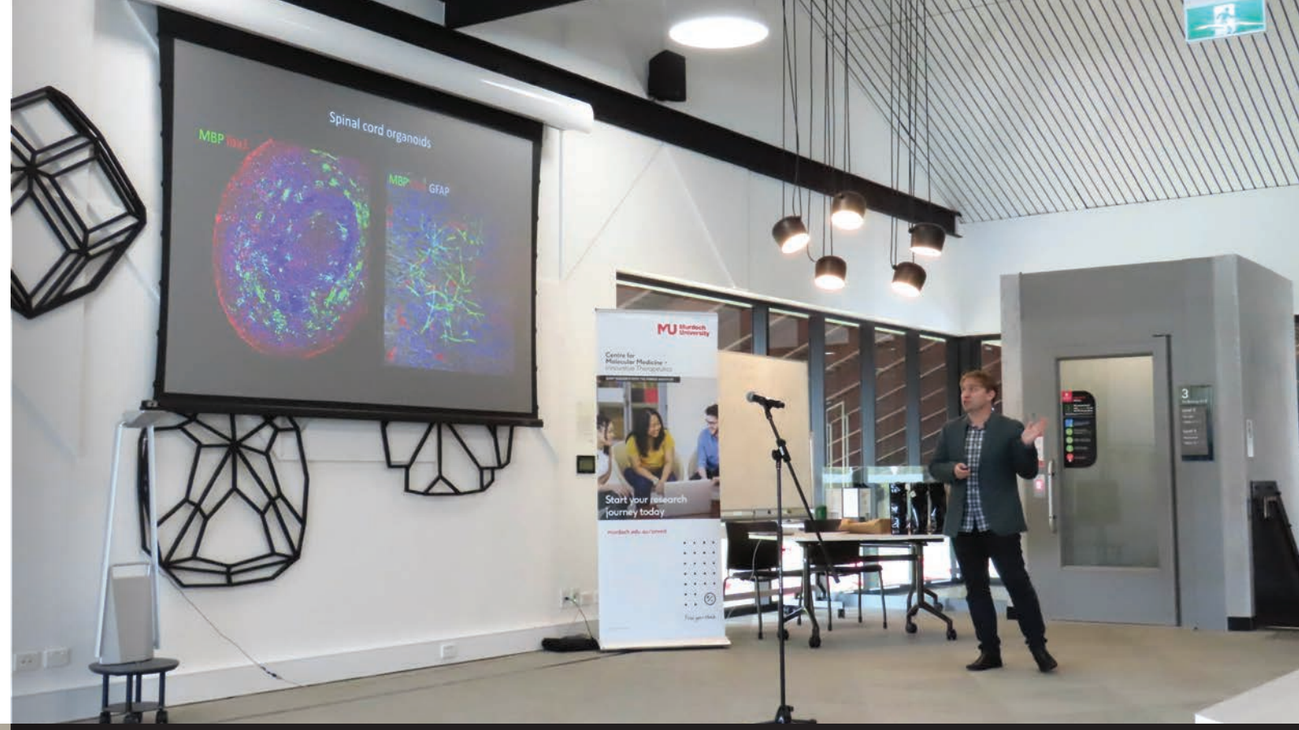
CMMIT had the pleasure of hosting a visit by the Hon Stephen Dawson MLC, Minister for Medical Research, in May to tour the Centre and discuss CMMIT's extensive research program - from genetics, genomics, and nucleic acid chemistry through to neurology, cognitive science, systems modelling and health economics.

CMMIT also welcomed the then newly appointed Murdoch Vice Chancellor, Professor Andrew Deeks for a tour of our facilities as well as many national and international guests throughout the year.

Parent Advisory Panel

With the help of the Western Australia Health Translation Network Community Involvement Program (CCI), CMMIT has formed a Parent Advisory Panel.

This enables us to tap into a community network we strive to support, as much of our research focuses on childhood diseases. Panel members have provided their perspectives on CMMIT's research allowing the involvement of consumers in all phases of CMMIT's research program on childhood disorders.



Below: CMMIT hosted the Hon Stephen Dawson, Minister for Medical Research.

Right top: CMMIT welcomed the newly appointed Murdoch University Vice Chancellor Andrew Deeks and Acting Deputy Vice Chancellor Research & Innovation, Professor Peter Davies.

Left bottom: Image from Community Conversation.

CMMIT Assembly

Sixty researchers and students participated in the CMMIT Assembly for a day of networking, collaboration and inspiration, with the aim to open up lines of communication and foster collaboration focusing on the bigger issues facing healthcare today.

Sponsored by the Perron Institute, the day brought together CMMIT staff and guests from other Murdoch Research Centres and beyond, for a cutting-edge program featuring guest speakers as well as overviews from each of CMMIT's research groups.

Highlights of the day included a keynote address 'Modelling sporadic ALS: from pipedream into pipeline of drugs' by internationally recognised neuroscientist Associate Professor Brad Turner, (Florey Institute of Neuroscience and Mental Health).

Medical Director of the Rare Care Centre based at the Perth Children's Hospital, Clinical Professor Gareth Baynam's presentation entitled '1 Billion Lifetimes of COVID' focused on the heavy toll rare diseases take, the impact on families and community, and the need for better resourcing if the burden of rare diseases is to be reduced.

Our People

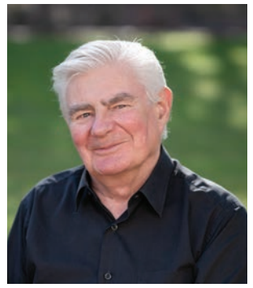
Management

PROFESSOR STEVE WILTON AO BSc PhD

Steve Wilton AO is Foundation Chair in Molecular Therapies at Murdoch University and has the dual roles of Director of CMMIT and Director of the Perron Institute. Internationally recognised with multiple awards, Steve jointly pioneered the use of ASOs to overcome some of the most common dystrophin gene defects causing Duchenne muscular dystrophy.

PROFESSOR ANTHONY AKKARI BSc PhD

Anthony Akkari is Foundation Chair in Industrial Pharmacogenetics at Murdoch University and Deputy Director of CMMIT. A pharmacogeneticist and neuromuscular geneticist by background, Professor Anthony Akkari spent over 18 years in the pharmaceutical industry working with GlaxoSmithKline, Eli Lilly and Cabernet Pharmaceuticals, where he focused on integrating genetic data into the drug development process.



PROFESSOR NORMAN PALMER BSc PhD

A biochemist by background and with extensive experience in research management in the university sector, Norman Palmer moved into the role of CMMIT Special Projects Director, after stepping down as CMMIT's Deputy Director in 2022. Norman was formerly Special Project Director at the Perron Institute, a position he held for over a decade.

MS JODIE WILLIAMSON

Jodie Williamson is CMMIT's Centre Manager. She has worked alongside Steve Wilton for over 10 years, in the process gaining an understanding of the special demands of managing staff and resources in a research environment. Her role is significant and multifaceted, encompassing grant and finance management, HR, strategy and operations.

Leadership Group

The Leadership Group provides oversight of CMMIT and its research program and is comprised of senior scientists as well as the Centre Manager and representatives of CMMIT's early career researchers and HDR students.

CMMIT has grown significantly since its establishment in 2019 and now involves a broad range of research fields. CMMIT's size and diversity means that it requires multiple levels of oversight. Whilst the Management group is responsible for day-to-day operations, the Leadership Group plays a crucial role in oversight, strategic planning and community outreach.

Left to right:
Professor Steve Wilton AO (Director CMMIT & Perron Institute)
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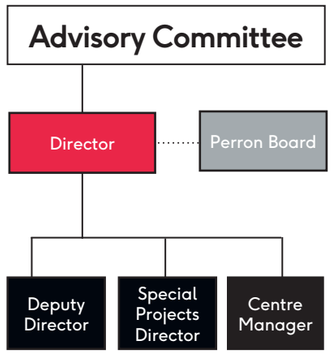

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Rare Voices Australia
Motor Neurone Disease WA
MSWA
Muscular Dystrophy WA
Myositis Association Australia
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Parkinson's WA
Racing for MNDi
Sock it to Sarcoma
Stroke WA

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