



**Murdoch**  
UNIVERSITY



Working together  
to solve global  
health challenges  
through the precision  
medicine revolution

**Centre for Molecular  
Medicine and Innovative  
Therapeutics**



# A new frontier in modern medicine – the precision medicine revolution

## Foreword from the Centre's Director

**M**odern medical science has tended to employ a one-size-fits-all approach to the treatment of disease. The traditional dogma has been to develop drugs specific for a particular disease, and then to use these in the treatment of all patients afflicted with that disease.

While many patients respond positively to this approach, others show little benefit, or even worse, may have adverse side effects.

At the Centre for Molecular Medicine and Innovative Therapeutics, we are strongly committed to using innovative scientific tools to develop and measure the capacity of precision medicine to improve the health and wellbeing of patients.

An initial focus of much of the Centre's research work has involved identifying the 'genetic spelling mistakes' in an individual's genetic code and then designing precision, molecular therapies to "compensate or bypass" those mutations.

These precise approaches are tailored to the unique molecular and genetic makeup of individual patients, allowing clinicians to predict disease onset and its likely progression, drug responsiveness and to develop and adapt management for maximum patient benefit.

The Centre will have a clinical facility where patients affected by serious health conditions like multiple sclerosis, myositis and other neurological and blood disorders will benefit from the specialist knowledge and findings of researchers and specialist clinicians.

We will build on our international record of successful translation of research findings, including the commercialisation of new drugs that have extended and improved the lives of people with serious, incurable genetic diseases, and eased the burden on their families.

With such an approach, the Centre provides an opportunity for Murdoch University to emerge as a genuine leader in the field of precision medicine.

**Professor Steve Wilton,**

**Director, Centre for Molecular Medicine and Innovative Therapeutics**



## Foreword from the Vice Chancellor

**T**he Centre for Molecular Medicine and Innovative Therapeutics will pioneer tailored treatments for rare and complex human diseases, based on genetics research.

The Centre will build on the world-leading research by Perron Institute scientists, now based at Murdoch University, who have delivered drugs tailored to address individual genetic defects associated with Duchenne muscular dystrophy (DMD). This global breakthrough has significantly extended the lifespan and functional abilities of young patients with this debilitating condition.

A joint venture between Murdoch University and the Perron Institute, the Centre brings together researchers and specialist clinicians in a range of rare diseases as well as in rehabilitation.

The Centre's developments in precision diagnostics, treatment and rehabilitation will directly benefit patients through the discovery of new drugs and clinical applications and is an important initiative to achieve Murdoch's purpose to be a creative force for current and future generations.

This Centre personifies the power of collaboration and increases the capacity for Murdoch University and the Perron Institute to shape the future direction of medical research in Western Australia and beyond, and I am looking forward with anticipation to what new discoveries lie ahead.

I invite you to join us on this important journey of discovery.

**Professor Eeva Leinonen,**  
Vice Chancellor, Murdoch University



## Foreword from Professor Alan Robson AO CitWA, Chair, Perron Institute

**T**o be meaningful, health and medical research must deliver tangible results that directly improve the health and wellbeing of people living with debilitating conditions. There is an increasing global focus on translational health research that leads to deep and lasting advances in healthcare.

The Perron Institute continues to develop the scale and critical mass needed to tackle the bigger issues in medical research. Integral to this, we see collaboration with other research organisations in Western Australia and worldwide as being absolutely critical to advancing ours, and the State's research agenda.

Our partnership with Murdoch University over the past six years, culminating in the establishment of the Centre for Molecular Medicine and Innovative Therapeutics, has been a major catalyst for change. It has made it possible to expand the range of diseases under investigation, and to create new and exciting partnerships with industry.

The Perron Institute welcomes the establishment of the Centre as a joint venture with Murdoch University, and looks forward to engaging in research that has the potential to change millions of lives in a truly significant way.

**Professor Alan Robson,**  
Chair, Perron Institute



## Our Director

### Professor Steve Wilton

**B**orn in Adelaide, Professor Steve Wilton is Director of the Perron Institute, Western Australia's longest established medical research institute. He is also Foundation Professor in Molecular Therapy at Murdoch University, where he co-leads the Molecular Therapy Research Group with long time colleague Professor Sue Fletcher.

The major focus of Professor Wilton's research has been the development of antisense oligonucleotide (AON)-based drugs for the treatment of Duchenne muscular dystrophy (DMD). Professors Wilton and Fletcher were responsible for the breakthrough discovery that AONs are able to overcome the genetic defect underlying DMD and delay the onset of the loss of the ability to walk that is the hallmark of the disease. One AON developed by Professors Wilton and Fletcher at the Perron Institute, Exondys-51, was granted accelerated approval by the US Food and Drug Administration (FDA) for use in patients in 2016, and two other AONs are in advanced clinical trials and expected to be submitted to the FDA in 2019/2020.

In recognition of the success of AONs in the treatment of DMD, Professors Wilton and Fletcher received the Western Australia Innovator of the Year Award (2012) and Eureka Prize for Medical Research Translation (2013). More recently, Professor Wilton received the LabGear Australia Discovery Science Award from the Australian Society for Biochemistry and Molecular Biology. Professor Wilton is past President of the Australian Gene Therapy Society.

# About the Centre

**The Centre for Molecular Medicine and Innovative Therapeutics (CMMIT) forms part of Murdoch University's and the Perron Institute's enduring commitment to excellence in health and medical research.**

**The Centre's goal is to undertake innovative and translational research that addresses urgent and complex issues facing healthcare today.**





## The purpose of establishing the Centre is three-fold:

1

To undertake cutting edge research that directly impacts on the health and wellbeing of people living with debilitating conditions

2

To focus on taking findings from the laboratory and clinic and translating these into commercial products, such as new drugs and diagnostics or improvements in clinical practice and health policy

3

To foster collaboration across the Centre's research groups with the goal of increasing the scope and quality of the Centre's research

## The changing pattern of disease

The last century has witnessed a remarkable transformation in human health. The average life expectancy in countries like Australia has increased by around 30 years since 1900, in part through better hygiene, immunisation and antibiotics lessening the burden of infectious diseases.

As our population ages, the major threat facing society has moved away from infectious diseases towards chronic diseases such as cancer, heart disease, stroke, diabetes and dementia. These changes in disease patterns over time have required medical science to evolve, a process that has culminated in the increasing application of the principles of precision medicine to a diversity of diseases.

The intellectual underpinning for the CMMIT is a deep focus on precision medicine. Our belief is that precision medicine has the potential to transform healthcare on a scale equivalent to the way antibiotics transformed the fight against infectious diseases.

To this point, the Centre's research focus has been on rare genetic disorders and on the use of antisense oligonucleotides (AONs) in the treatment of Duchenne molecular dystrophy (DMD) and other diseases. This research has a strong translational focus that has led to the development of novel drugs for DMD, including one that has now been approved for use in patients by the US Food and Drug Administration.

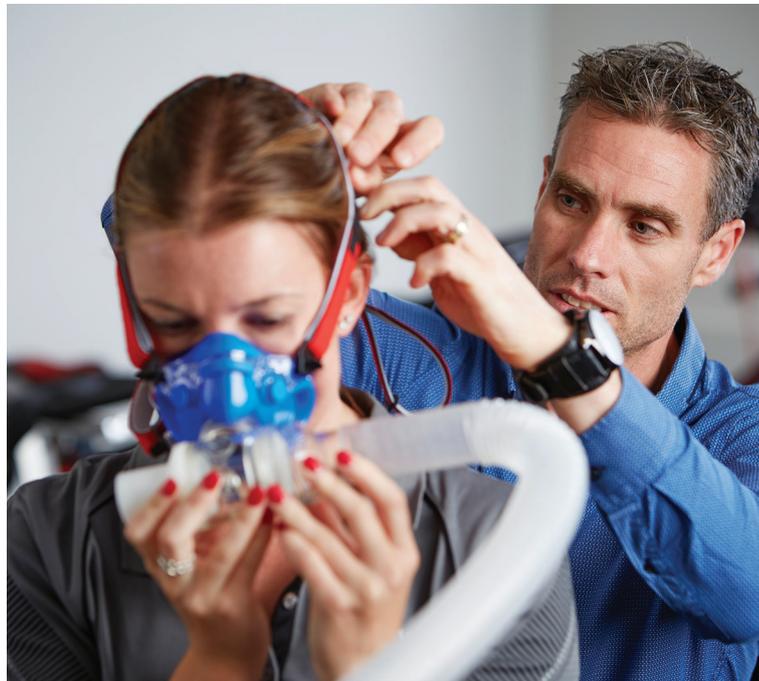
Over the last few years, the Centre's research and therapeutic advances have grown to include a broader range of diseases, including other types of muscular dystrophy, motor neurone disease, Parkinson's disease, collagenopathies, Marfan syndrome and eye disorders. The Centre's clinical research capacity has also grown, with a focus particularly on multiple sclerosis, myositis and blood disorders. Precision medicine has multiple components, and the creation of the Centre adds an exciting new dimension with the involvement of researchers in the fields of cognition, biomechanics and physiology, allowing the principles of precision medicine to improve the effectiveness of rehabilitation.

**We see our Centre as an engine to drive innovation and commercialisation at Murdoch University in the field of precision medicine.**

**Economic imperative for innovation through research commercialisation**

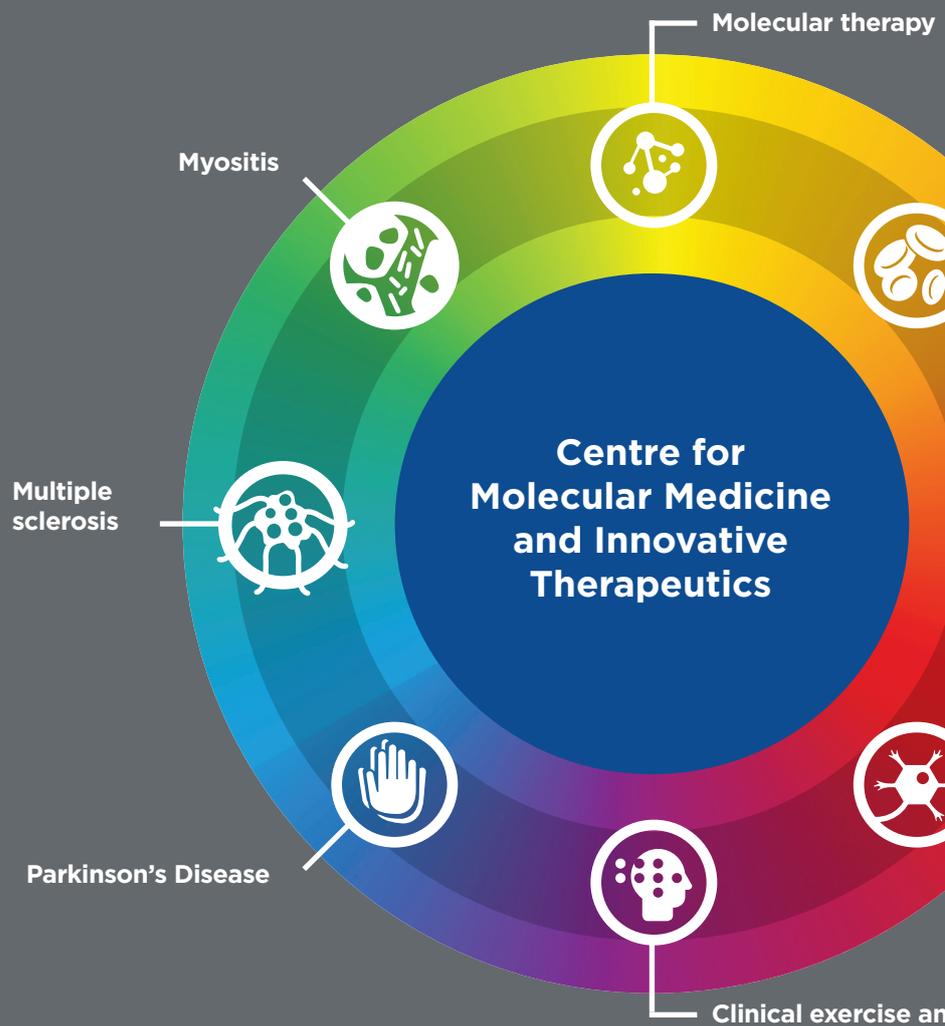
Australia performs strongly in terms of research outputs contributing 3.9 per cent of the world's research publications from 0.3 per cent of the world's population. However, by international standards it underperforms in terms of translating research into commercial outcomes. Against this background, our Centre is strongly focused on accelerating the uptake of findings from the laboratory and clinic into commercial outcomes, particularly new drugs and diagnostics that directly and immediately benefit patients. As such, the goals of the Centre closely align with government priorities at the State and Commonwealth levels.

Consequently, we see our Centre as an engine to drive innovation and commercialisation at Murdoch University in the field of precision medicine.



**Creating a Centre that is greater than the sum of the parts**

Our goal is to make the whole greater than the sum of its parts by breaking down discipline barriers and bringing researchers together in an integrated Centre. We seek to create critical mass leading to higher quality research outputs and outcomes.





## Research aimed at making a difference

**Our Centre's research is diverse and is spread across eight broad research areas:**

**Molecular Therapy** – Professors Steve Wilton and Sue Fletcher focusing on developing genetic drugs to treat Duchenne muscular dystrophy (DMD) and other inherited disorders. Currently they are looking at over 50 different diseases.

**Precision Nucleic Acid Therapeutics** – Dr Rakesh Veedu leads the development of novel therapeutic molecules that facilitate target-specific delivery of drugs or diagnostics to specific disease sites in the body. This includes solid cancers, Type-2 diabetes and diabetic retinopathy, and neurological diseases, including Alzheimer's disease.

**Motor neurone disease** – Professor Anthony Akkari and team are investigating new approaches to the treatment of the fatal motor neurone disease (MND), a disease affecting 350,000 people worldwide.

**Parkinson's Disease** – Professor Sulev Kõks is newly-appointed to the Centre and will explore the genetics and molecular pathology of Parkinson's disease - with the goal of improving the precision clinical management of patients.

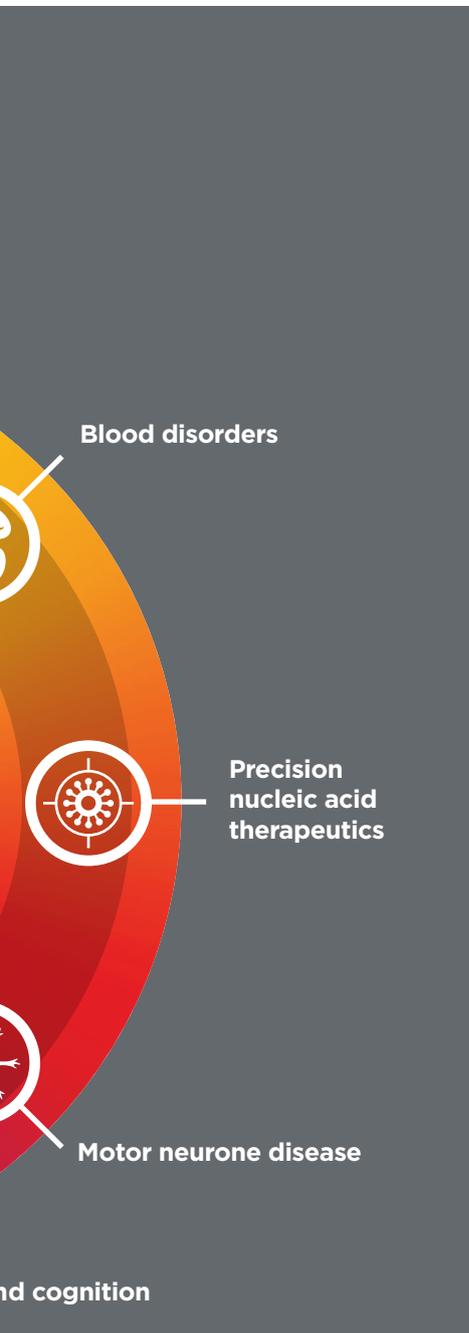
**Clinical Exercise and Cognition** – Associate Professor Tim Fairchild, Dr Alasdair Dempsey, Dr Hakuei Fujiyama, Dr Yvonne Learmonth, Associate Professor Jeremiah Peiffer, Dr Sean Muller and Dr Ann-Mareé Vallence are leading research into biomechanics, physiology, metabolism and cognition. Their expertise will be invaluable when precisely assessing the benefits during the development of personalised therapies.

**Multiple Sclerosis** – Clinical Professors Allan Kermode and Bill Carroll are exploring the clinical, laboratory, radiological, and genetic aspects of multiple sclerosis, which affects around 23,000 Australians.

**Blood disorders** – Professor Ross Baker is working to address the unmet needs of patients who die or suffer from blood disorders. He is also investigating patients who are susceptible to thrombosis and bleeding in response to anticoagulants or drugs that affect platelet function.

**Myositis** – Professors Merrilee Needham and Frank Mastaglia are investigating the treatment, genetics and immuno-pathology of immune-mediated myositis,

**The creation of the Centre will be the catalyst for the development of a network of collaborations linking the Centre's research groups and creating links with other researchers at Murdoch University, the Perron Institute, around Australia and the world.**



**The creation of the Centre will be the catalyst for the development of a network of collaborations linking researchers at Murdoch, the Perron Institute and around the world.**





**“It’s far more important to know what person the disease has than what disease the person has.”**

Hippocrates,  
circa 400 BC

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