Murdoch University

Centre for Molecular Medicine + Innovative Therapeutics

JOINT RESEARCH WITH THE PERRON INSTITUTE

Commercial Services

A world-class service in design and pre-clinical testing of antisense oligonucleotide and other nucleic acid-based therapeutics for industry and academia

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Tap into the unique expertise of one of the world's most successful research centres in the field of antisense oligonucleotide (ASO) therapeutics.

The originator of three FDA-approved ASOs for the treatment of Duchenne muscular dystrophy plus multiple patents for other diseases, CMMIT is at the forefront of the worldwide search for ASO therapeutics for both inherited and acquired disorders.

CMMIT's ASO design and pre-clinical cell-based development platform offers our clients and partners worldwide access to over a quarter of a century's experience in the design and development of ASOs tailored to your organisation's specific needs.

We offer a fast and reliable service that provides high-quality solutions in your organisation's quest for therapeutically-effective ASOs.

ABOUT CMMIT

Based in Perth, Western Australia, CMMIT is a joint research centre of Murdoch University and the Perron Institute, Western Australia's oldest medical research institute.

CMMIT's area of focus is precision medicine, particularly ASOs and other nucleic acid-based therapeutics. CMMIT involves researchers from a broad range of fields from genetics, genomics, and nucleic acid chemistry through to neurology, cognitive science, systems modelling and health economics as we believe that this breadth of research expertise adds to our unique capacity to translate research findings into real-world outcomes in the clinic, a process further enabled by the involvement of consumers in all phases of CMMIT's research program.

CMMIT - OUR STORY

CMMIT's research on ASOs began over a quarter of a century ago with ground-breaking research proving the ability of ASOs to manipulate pre-mRNA processing in Duchenne muscular dystrophy. Their research showed that a still functional but internally truncated form of dystrophin is produced rather than a non-functional form. This provided the foundation for Sarepta Therapeutics to develop three ASOs targeting different subclasses of mutations in the dystrophin gene – Exondys 51, Vyondys 53 and Amondys 45 – through to the point of FDA, thereby providing clinicians with the tools to treat almost 30% of Duchenne cases.

The success of the ASOs for Duchene muscular dystrophy has sparked a major expansion in CMMIT's research, resulting in over 50 different diseases currently being under investigation as potential targets for treatment with ASOs. Our aim is to continue to develop and patent ASO therapeutics for an increasing range of disorders. CMMIT's experience in ASO design coupled with our expertise in nucleic acid chemistry puts it at the forefront of the ASO field internationally and provides the platform upon which our ongoing research is based.

Aside from grant-funded discovery research on ASOs, CMMIT works increasingly with commercial and academic clients and partners to help them design and validate ASOs.

OUR SERVICES

CMMIT offers a range of services to clients and partners from both academia and industry. These services include:

- Genomics and in silico analysis
- ASO design
- Design optimisation
- Choice of chemistries
- In vitro testing of ASO efficacy, including in relevant induced pluripotent stem cells (IPSCs)

CMMIT has in place a well-tested ASO design and pre-clinical development platform that provides clients and partners worldwide with cutting-edge support in the design and development of ASOs tailored to your organisation's specific needs.

We offer a world-class, fast and reliable service that provides high-quality solutions in your organisation's quest for therapeutically-effective ASOs.

CONTACT US

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+61 8 9360 6088

OUR SCIENCE LEADERS

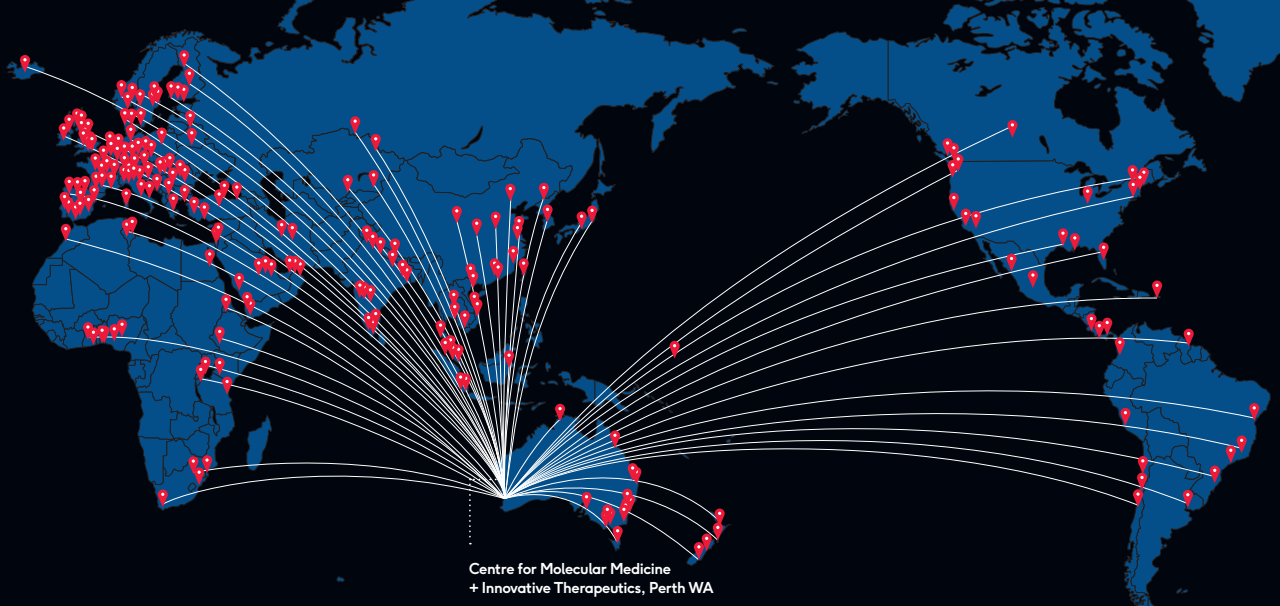
CMMIT's core capabilities in the fields of genetics, genomics and nucleic acid therapeutics involves 40 scientists and PhD students. Leadership is provided by internationally-recognised senior scientists with expertise in ASO design and commercialisation, nucleic acid chemistry and genomics:

PROFESSOR STEVE WILTON AO BSc PhD & **DR MAY AUNG-HTUT** BSc PhD
Steve is the Director of the Perron Institute and Founding Director, now Deputy Director of CMMIT. In collaboration with Sue Fletcher, Steve pioneered the use of ASOs to overcome some of the most common dystrophin gene defects causing Duchenne muscular dystrophy. Over a research journey spanning more than two decades, Steve took the idea of using ASOs to treat Duchenne from the laboratory through to clinical application, through a partnership with the US pharmaceutical company, Sarepta Therapeutics. Together May and Steve head CMMIT's Molecular Therapy group, which is designing and evaluating novel mechanisms in the use of ASOs for more than 50 different diseases and has generated multiple patents. Recent interests include multiple sclerosis, muscular dystrophy, adult Pompe's diseases and ultra-rare genetic disorders.

PROFESSOR ANTHONY AKKARI BSc PhD **& DR LOREN FLYNN** BSc PhD
Anthony and Loren head CMMIT's Motor Neurone Disease/ALS Genetics and Therapeutics Research group. A pharmacogeneticist and neuromuscular geneticist by background, Anthony spent many years in the pharmaceutical industry with GSK, Eli Lilly and Cabernet Pharmaceuticals, where he focused on integrating genetic data into the drug development process. Anthony is Director of CMMIT and Chief Scientific Officer of Black Swan Pharmaceuticals (BSP), a North Carolina-based ALS drug development company. Loren is a molecular geneticist with 14 years of expertise in developing antisense therapies for modulating gene expression in rare genetic and neurodegenerative disorders, including ALS, and PD. Loren is the Director of Therapeutic Development at BSP and leads the development of the ALS and PD antisense portfolio from early discovery to pre-clinical evaluation.

ASSOCIATE PROFESSOR RAKESH VEEDU MSc PhD MRACI
Rakesh heads CMMIT's state-of-the-art DNA, RNA and chemically-modified oligonucleotide synthesis facility and is Managing Director of the Perth-based spinoff company, SynGenis, a supplier of research-grade nucleic acids for use in industry and academia. A graduate of the University of Queensland and Griffith University, Rakesh worked for several years at the University of Southern Denmark under the mentorship of Professor Jesper Wengel.

PROFESSOR SULEV KOKS MD PhD
Sulev heads CMMIT's Genetic Epidemiology research group. A PhD and MD graduate from the University of Tartu in Estonia, Sulev completed his postdoctoral studies in genomics at King's College, London. His current research focuses on understanding the molecular mechanisms underlying neurodegenerative disorders, including Parkinson's disease. Sulev's research interests are broad and include psoriasis, psoriatic arthritis, osteoporosis, osteosarcoma and metabolic syndrome. Sulev is a highly published researcher and an active member of several international research consortia.



CMMIT collaborates with:

- **ALL** Western Australian universities, research institutes & major hospitals
- **NATIONALLY** with 104 institutions across all Australian States & Territories
- **INTERNATIONALLY** - with 1,194 institutions in 92 countries

TESTIMONIAL

Professor Lorna Harries
Chief Scientific Officer & Co-founder of SENISCA

SENISCA are an early-stage biotech spin out company linked to the University of Exeter. We are focused on the development of oligonucleotide drugs for age-related diseases, based on our discovery of a new and druggable hallmark of cellular ageing. Our technology is based on the restoration of correct splicing regulation in ageing cells. Splicing factor expression declines with ageing but is necessary for molecular stress resilience and avoidance of cellular senescence. We have been working with Professor Steve Wilton and his team to design and evaluate oligonucleotide interventions capable of attenuating the expression of some of the 'master control' genes that regulate splicing factor expression for therapeutic benefit.

> "We have found the input of the Murdoch team to be invaluable. It has been a real benefit to SENISCA for us to be able to draw on decades of expertise in this space to aid in the design and optimisation of our oligonucleotides, and to provide an evaluation of target engagement in different cell types."

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