Personalised Medicine Centre

Murdoch University | perron institute

South Street, Building 390

Molecular Therapy Laboratory

Student Brochure

2025 - 2026

moleculartherapy@murdoch.edu.au

Research opportunity:	Honours	X	Masters		PhD	
Project title:	Identification of Therapeutic Compounds for Childhood Dementia					

Short project description & main objectives:

Childhood dementia is caused by more than 70 rare genetic diseases. Symptoms can include memory loss, confusion, developmental regression, seizures, and difficulties with movement and communication. Sadly, it also affects the child's ability to recognise family and friends. Half of the children with dementia die by the age of 10, and there is currently no cure, though research and supportive therapies aim to improve quality of life.

Our laboratory has three decades of experience in designing and evaluating a class of drugs known as antisense oligomers (ASOs) for both rare and common diseases. We are now developing treatments for childhood dementia using ASOs.

This project aims to:

1. **Modify the ASO identified from the preliminary screen to improve the efficacy of the drug**
2. **Compare the lead ASOs to identify the most efficient and safe chemistry**

This project will involve:

- Mammalian cell culture
- Delivery of ASOs (Transfection)
- RNA extraction
- RT-PCR
- Live cell imaging

This project will be conducted in a PC2 facility at the Personalised Medicine Centre, Murdoch University.

Principal supervisor:	Dr May Aung-Htut
Other supervisors:	Dr Jessica Cale
Contact details for further information:	m.aung-htut@murdoch.edu.au
Closing date for applications:	July 2025
Start & finish date of project:	S2 2025 – S1 2026
Available part-time?	No
Available to international students?	Yes

If applicable:

Research centre/group:	Personalised Medicine Centre, Molecular Therapy Laboratory
Desired background of applicants:	Gene expression
Additional funding/scholarship provided:	NA
Other benefits:	
Extra Comments:	

Research opportunity:	Honours	X	Masters		PhD	
Project title:	Exploring Antidotes for Drug Toxicity					

Short project description & main objectives:

Our laboratory has three decades of experience in designing and evaluating a class of drugs known as antisense oligomers (ASOs) for both rare and common diseases. We want to explore a way to counteract unexpected adverse events in patients when an ASO is administered. We will design antidotes based on our current knowledge of the drug mechanisms and assessed them in the human cell lines.

This project aims to:

1. **Design antidotes for ASO drugs**
2. **Assess the potential antidotes in human cells for their ability to prevent/stop selected ASO drug toxicity.**

This project will involve:

- Mammalian cell culture
- Delivery of ASOs (Transfection)
- RNA extraction
- RT-PCR
- Live cell imaging

This project will be conducted in a PC2 facility at the Personalised Medicine Centre, Murdoch University.

Principal supervisor:	Dr May Aung-Htut
Other supervisors:	Dr Jessica Cale
Contact details for further information:	m.aung-htut@murdoch.edu.au
Closing date for applications:	July 2025 or Jan 2026
Start & finish date of project:	S2 2025 – S1 2026, S1 2026 – S2 2026
Available part-time?	No
Available to international students?	Yes

If applicable:

Research centre/group:	Personalised Medicine Centre, Molecular Therapy Laboratory
Desired background of applicants:	Gene expression
Additional funding/scholarship provided:	NA
Other benefits:	
Extra Comments:	

Research opportunity:	Honours	X	Masters		PhD	
Project title:	Establishing an Age-Appropriate Neuronal Cell Model to Study Age Related Neurological Disorders					

Short project description & main objectives:

Do you want to be part of a team that's helping to develop new medicines for serious diseases like dementia and Alzheimer's? Our lab has been working on a type of medicine called *antisense oligomers* (ASOs) for over 30 years, and some of these drugs are already helping children with muscle diseases like Duchenne Muscular Dystrophy. Now, we're focusing on brain-related conditions—and we need your help!

In this project, you will help build a simple, brain-like cell model in the lab. These cells are made by "reprogramming" ordinary skin cells (called fibroblasts) into neuron-like cells using a special mix of small molecules. This method keeps the "age" of the cells, which helps us better study diseases that happen later in life, like Alzheimer's Disease.

Once we have these neuron-like cells, you'll test how well ASOs work by measuring their effects on gene activity and proteins linked to aging and brain disease. This project will take place in a certified PC2 lab at the Personalised Medicine Centre, Murdoch University.

This project aims to:

1. Create a brain cell model from human skin cells using small molecules.
2. Test how well ASOs enter the cells and affect gene expression.
3. Measure changes in proteins linked to age-related brain conditions.

You'll learn hands-on skills in:

- Growing human and neuron-like cells in the lab (cell culture).
- Delivering ASOs into cells (transfection).
- Extracting RNA and checking gene expression (RT-PCR, qPCR).
- Using lab techniques like immunohistochemistry and ELISA to study proteins.

General Requirements for all Students:

- Strong time management and organisation skills to follow the experimental schedule.
- Careful attention to detail and willingness to follow safety and Good Laboratory Practice (GLP) standards.
- Ability to work independently and as part of a supportive team.
- Interest in medical research, genetics, neuroscience, or biotechnology.

Principal supervisor:	Dr Kelly Martinovich
Other supervisors:	Dr May Aung-Htut Mr Aidan Murphy
Contact details for further information:	Kelly.Martinovich@murdoch.edu.au
Closing date for applications:	July 2025
Start & finish date of project:	S2 2025 – S1 2026
Available part-time?	No
Available to international students?	Yes

If applicable:

Research centre/group:	Personalised Medicine Centre, Molecular Therapy Laboratory
Desired background of applicants:	Gene expression, Molecular Biology
Additional funding/scholarship provided:	NA
Other benefits:	
Extra Comments:	

Research opportunity:	Honours	X	Masters		PhD	X
Project title:	The Development of Novel Tailored Therapeutics for People with Familial Hypercholesterolemia					

Are you interested in genetics, heart health, and cutting-edge therapies?
This project focuses on familial hypercholesterolemia (FH)—a common inherited condition that causes dangerously high levels of cholesterol from a young age. People with FH often develop heart disease early in life, sometimes as young as their teens.
FH is usually caused by mutations in the *LDLR* gene, which is responsible for clearing "bad cholesterol" from the blood. Our lab is working on a new way to help these patients by using *antisense oligonucleotides* (ASOs)—or what we like to call *gene patches*. These patches work a bit like molecular correction tape, covering up the faulty section of the gene so that the rest can still do its job. You'll be part of a research effort to develop these gene patches and test how well they work in cells—and possibly in animal models too. This project will be conducted in a certified PC2 lab at the Personalised Medicine Centre, Murdoch University.

Overall project aims:

1. Create cell models using samples from people with FH.
2. Design and test gene patches (ASOs) tailored to patient mutations.
3. Deliver ASOs into cells and check gene activity using mRNA analysis.
4. Test whether the gene patches improve cholesterol uptake.
5. Explore how these ASOs work in small animal models (optional).

You'll learn practical skills in:

- Mammalian cell culture and liver organoid culture
- Gene patch delivery (transfection)
- Gene expression analysis (RT-PCR, qPCR)
- Histology, immunohistochemistry, and western blotting
- Live cell imaging and flow cytometry
- Working with small animals (optional)

General requirements for all students:

- Strong time management and attention to detail
- Good organisation and note-keeping to track experiments
- Willingness to follow safety procedures and Good Laboratory Practice (GLP)
- Enthusiasm for learning and working in a collaborative team
- Interest in molecular biology, genetics, medicine, or biotechnology

Principal supervisor:	Dr Kelly Martinovich
Other supervisors:	Dr May Aung-Htut
Contact details for further information:	Kelly.Martinovich@murdoch.edu.au
Closing date for applications:	July 2025/ ongoing
Start & finish date of project:	S2 2025 – S1 2026 / ongoing
Available part-time?	No
Available to international students?	Yes

If applicable:

Research centre/group:	Personalised Medicine Centre, Molecular Therapy Laboratory
Desired background of applicants:	Gene expression, Molecular Biology
Additional funding/scholarship provided:	NA
Other benefits:	
Extra Comments:	For PhD applicants, we will support you in applying for scholarships at Murdoch University and other sources as applicable.

Research opportunity:	Honours	X	Masters		PhD	
Project title:	Evaluating New Chemistries to Improve Fibrillin-1 ASOs					

Short project description & main objectives:

Marfan syndrome is a dominant connective tissue disorder cause by mutations in the fibrillin-1 (*FBN1*) gene that disrupt the formation of fibrillin-1 microfibrils. Marfan syndrome is characterised by eye, bone, skin and heart abnormalities, and while life expectancy of affected individuals has increased due to advancement in surgical interventions, there is still no effective therapies.

Our laboratory has three decades of experience in designing and evaluating a class of drugs known as antisense oligomers (ASOs) for both rare and common diseases. Our preliminary data shows that ASOs can alter the exon structure of *FBN1* mRNA transcripts to re-establish the production of fibrillin-1 microfibrils.

This project aims to:

1. **Assess the ASOs identified from preliminary research as a newer chemistry to determine the most efficient and safe option.**
2. **Assess the effect of lead ASOs as various chemistries on fibrillin-1 microfibril formation**

This project will involve:

- Mammalian cell culture
- Delivery of ASOs (Transfection)
- RNA extraction
- RT-PCR
- Immunofluorescence staining

General requirements for all students:

- Strong time management and attention to detail
- Good organisation and note-keeping to track experiments
- Willingness to follow protocols and procedures
- Enthusiasm for learning and working as a team

This project will be conducted in a PC2 facility at the Personalised Medicine Centre, Murdoch University.

Principal supervisor:	Dr Jessica Cale
Other supervisors:	Dr May Aung-Htut
Contact details for further information:	jessica.cale@murdoch.edu.au
Closing date for applications:	July 2025 or Jan 2026
Start & finish date of project:	S2 2025 – S1 2026, S1 2026 – S2 2026
Available part-time?	No
Available to international students?	Yes

If applicable:

Research centre/group:	Personalised Medicine Centre, Molecular Therapy Laboratory
Desired background of applicants:	Gene expression, Molecular Biology
Additional funding/scholarship provided:	NA
Other benefits:	
Extra Comments:	

Research opportunity:	Honours	X	Masters		PhD	
Project title:	Urine-derived Stem Cells as Non-Invasive Proxies for Neuronal Transcriptomes in Rare Disease Diagnosis					

Short project description & main objectives:

Urinary stem cells (USCs) are a promising non-invasive cell source for transcriptomic analysis in patients with rare or severe diseases where neuronal tissue biopsy is not feasible. Establishing whether USCs can serve as a proxy for neuronal cells could provide a valuable tool for diagnosis and therapeutic research, especially in paediatric and neurodegenerative conditions.

This project will investigate the transcriptomic similarity between USCs and neuronal cell types by utilising long-read PacBio RNA sequencing data. Specifically, it will quantify isoform-level overlap, assess the presence of cell-type-specific isoforms, and apply computational analyses such as clustering and dimensionality reduction to evaluate overall transcriptomic similarity.

This project aims to:

1. **To quantify isoform overlap between USC, induced pluripotent stem cells (iPSC), and neural progenitor cells (NPC) transcriptomes using long-read sequencing data.**
2. **To identify and characterise isoforms unique to neuronal cells and evaluate their presence or absence in USCs.**
3. **To assess overall transcriptomic similarity using clustering and dimensionality reduction techniques**

This project will involve:

1. Long-read RNA-seq analysis (PacBio IsoSeq)
2. Transcript annotation and isoform quantification
3. Dimensionality reduction and clustering (e.g. UMAP, PCA)

This project will be computational in nature and will use existing PacBio datasets from USC, iPSC, and NPC lines. Analysis will be conducted using high-performance computing resources with support from the supervisors.

Principal supervisor:	Dr Anu Sooda
Other supervisors:	Dr Jessica Cale
Contact details for further information:	a.sooda@murdoch.edu.au
Closing date for applications:	Jan 2026
Start & finish date of project:	S2 2025 – S1 2026, S1 2026 – S2 2026
Available part-time?	No
Available to international students?	Yes

If applicable:

Research centre/group:	Personalised Medicine Centre, Molecular Therapy Laboratory
Desired background of applicants:	Gene expression, bioinformatics
Additional funding/scholarship provided:	NA
Other benefits:	
Extra Comments:	

Research opportunity:	Honours	X	Masters		PhD	
Project title:	Dissection of Hidden Layers of Microexon Splicing Signatures in Neurological Disorders					

Short project description & main objectives:

Microexons, small exons ranging from 3 to 27 nucleotides, have recently been recognised as key regulators of gene expression, particularly in neuronal function. Despite their small size, these exons can profoundly impact protein structure and cellular processes. Dysregulation of microexon splicing has been implicated in various neurological disorders, including autism spectrum disorder, epilepsy, and others. However, the role of microexons in these diseases remains largely unexplored. This gap in understanding is partly due to the challenges in detecting these low abundance splicing events, as they are often overlooked or misannotated in standard genome annotations. As a result, the potential contribution of microexons to disease mechanisms has yet to be fully appreciated.

This project aims to develop a computational pipeline specifically designed to identify and analyse microexon splicing events in RNA-seq data from neurological disorder cohorts. This pipeline will focus on detecting low abundance splicing events that are often missed by conventional annotation tools.

Objectives:

1. Develop computational pipeline to detect microexon splicing events in RNA-seq data from neurological disease cohorts.
2. Quantify and compare microexon inclusion/exclusion patterns across disease states.
3. Experimentally validate a subset of predicted microexons using RT-PCR and sequencing to assess their regulatory role in disease.

This project will involve:

- Downloading public RNA-seq datasets
- Building a pipeline using available bioinformatic tools
- RT-PCR and Sanger sequencing

This project will be computational in nature and analysis will be conducted using high-performance computing resources with support from the supervisors.

Principal supervisor:	Dr Anu Sooda
Other supervisors:	Dr May Aung-Htut
Contact details for further information:	a.sooda@murdoch.edu.au
Closing date for applications:	July 2025 or Jan 2026
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Available part-time?	No
Available to international students?	Yes

If applicable:

Research centre/group:	Personalised Medicine Centre, Molecular Therapy Laboratory
Desired background of applicants:	Gene expression, bioinformatics, molecular biology
Additional funding/scholarship provided:	NA
Other benefits:	
Extra Comments:	