



# LAB-GROWN 'TINY HEARTS': DEVELOPMENT OF AN EPICARDIAL LINEAGE TRACING HUMAN PLURIPOTENT STEM CELL LINE TO IDENTIFY NEW CARDIAC THERAPEUTICS

## CELL AND GENE THERAPIES

Cardiovascular disease is a major cause of death in Australia, responsible for one in four of all deaths. Genetic heart disease and inherited heart conditions like cardiomyopathies, are incredibly difficult to understand and treat due to our limitations in disease modelling. Unlike cancer, cardiovascular disease, and heart failure, have very limited treatment options. A collaboration between QIMR Berghofer, Murdoch Children's Research Institute (MCRI) and the Royal Children's Hospital has developed lab-grown 'tiny hearts' to address this gap and discover new therapeutics for patients. These 'tiny hearts' created by QIMR Berghofer's **Prof. James Hudson** and fellow scientists mimic human heart muscle and behave like a genuine adult heart. The true breakthrough of this collaboration is the acceleration of the maturation process of the organoids, with the ability to make hundreds per week.

The team initially received support through a Technical Feasibility Assessment Voucher from Therapeutic Innovation Australia, which enabled detailed translation and manufacturing discussions with Cell Therapies Pty Ltd. These discussions identified a pathway for clinical development of the novel heart cell patch. This foundational support helped secure a Pipeline Accelerator voucher from Phenomics Australia which played a fundamental role

in enabling access to the iPSC Derivation and CRISPR Gene Editing Facility, at MCRI.

The early support of both the Pipeline Accelerator and TFA scheme has been instrumental in creating a solid platform for translating their findings into larger drug discovery and disease modelling projects.

With these human cardiac organoids, researchers can be confident that the translation from petri dish to drug development will progress faster. Technologies like these allow researchers to screen new therapeutics and make it possible on a larger scale than before. The team has already had positive responses to various drugs that have known cardiac effects in the clinic and will begin larger screening projects to improve Australia's ability to understand and treat cardiac disease in the future.



"The support of the voucher scheme enabled us to generate a novel line, which enabled us to perform lineage tracing of stromal cells in human cardiac organoids for the first time. This provided really important information to show that these cells resemble native cardiac cells. We hope to now extend these findings and discover new therapeutics targeting the stromal cells for heart failure." **Prof. James Hudson.**

### Impact:



TRL 1

TRL 2

TRL 3

TRL 4

TRL 5

TRL 6

TRL 7

TRL 8

TRL 9