Production of pluripotent cell derived cardiomyocytes.

ADVANCING CARDIAC REGENERATIVE MEDICINE THROUGH GMP-READY STEM CELL THERAPY

CELL AND GENE THERAPIES

Heart failure remains a major global health challenge, with limited treatment options beyond heart transplantation. **Prof James Chong** (University of Sydney), **Dr. Andrew Prowse** (University of Queensland), and **Dr. Steve Dingwall** (Australian Institute for Bioengineering and Nanotechnology) are leading efforts to commercialise pluripotent stem cell-derived cardiomyocytes (PSC-CMs) as a scalable and safe alternative to transplantation. By refining production methods, this initiative aims to provide a reliable, high-quality cell therapy product to treat patients in heart failure.

The Pipeline Accelerator awards have been key enablers in leveraging a $4.9M MRFF grant, allowing the project team to access Q-Gen Cell Therapeutics and Sydney Cell and Gene Therapy while accelerating progress toward clinical translation.

At Q-Gen Cell Therapeutics, the team received hands-on training in GMP standards, environmental monitoring, and quality management, enabling a pilot GMP production of PSC-CMs. This collaboration refined production protocols, ensured GMP compliance, sourced materials, managed documentation, and conducted batch testing—key for Therapeutic Goods Administration compliance.

The team were then able to transfer PSC-CM batches to Sydney Cell and Gene Therapy in order to establish GMP-compliant formulation, clinical delivery protocols, and process execution under a suitable quality management system— a critical step for advancing the PSC-CM program toward Phase I clinical trials.

This project has successfully established a master cell bank, demonstrated proof-of-concept for GMP-compliant PSC-CM production, and progressed toward a pre-regulatory safety data package. Since receiving the TIA voucher, the team has secured an additional $816,000 grant from the Australian Stroke and Heart Research Accelerator. Additionally, the initiative has fostered new collaborations and positioned Australia as a leader in cardiac regenerative medicine. With clinical trials and commercialisation on the horizon, this work is set to transform heart failure treatment.

> "Translating our research to a clinical trial is a big gear change and we would not have been able to produce GMP compliant cells without our continued involvement with TIA's facilities" **Prof James Chong**

Impact:

TRL 1 | TRL 2 | TRL 3 | TRL 4 | **TRL 5** | **TRL 6** | TRL 7 | TRL 8 | TRL 9