Melbourne Biomedical Precinct.

Research Platform 05

Stem cells and regenerative medicine

Defining stem cells and regenerative medicine

Stem cells have the remarkable ability to become any of the hundreds of specialised cells that make up our bodies, from skin, kidney and bone cells through to neurons and red blood cells. Scientists have also figured out how to 'reprogram' specialised cells, such as a skin cell, and revert it to a stem cell. These 'induced pluripotent stem cells', called iPS cells, can then be coaxed back into becoming any of the other cell types in our bodies. Regenerative medicine is the application of stem cells to repairing the damage from injury or disease.

The Melbourne Biomedical Precinct is at the forefront of stem cell research in Australia and is uniquely positioned to capitalise on the coming age of regenerative medicine, with applications to tackle a range of rare and common diseases.

We are about to enter a new era of regenerative medicine, thanks to recent breakthroughs in stem cell science, particularly the ability to create 'induced pluripotent stem cells' (iPS cells). Melbourne Biomedical Precinct researchers are already world leaders in using these iPS cells and are progressing research in three broad areas.

The first is to better understand the causes of complex diseases. One example is inherited kidney disease, which can appear in very young children and result in kidney failure, blindness, hearing loss or bone abnormalities. For most patients, there is no way to prevent total kidney failure and there is little understanding of how to slow the process down. Melbourne Biomedical Precinct researchers have been able to create iPS cells from inherited kidney disease sufferers and then use them to grow miniature versions of their diseased kidneys, called 'organoids', in the lab. These kidney organoids are proving to be an incredibly powerful tool for understanding how the disease unfolds. Coupled with the latest genomic sequencing technology, researchers can zero in on the specific genetic mutations that cause the disease. This information will be invaluable for improving diagnosis and developing new treatments that might one day be able to cure this and many other diseases.

Clinicians will also soon be able to use the stem cells found in adult tissue and in cancers to develop truly 'personalised' treatments for a particular individual. So instead of a cancer patient trying several chemotherapy drugs to find the most effective one, the drugs can be tested in the lab on organoids produced from the patient's own cancer cells. This will enable clinicians to choose the optimal treatment for the patient.

The second area of research is investigating how to use stem cells to repair or replace cells damaged by injury or disease. This is already being done successfully for patients with leukaemia having a bone marrow transplantation. The hope is that by better understanding stem cells for other tissues, or by generating new tissue from iPS cells, we will be able to develop treatments for other serious diseases that affect hundreds of thousands of Australians, including heart disease, type 1 diabetes, Parkinson's disease, macular degeneration and rheumatoid arthritis.

Stem cells don't have to be applied directly to regenerate tissue. They can be used in the laboratory to identify the triggers that will prompt the body to heal itself. For example, we know the hearts of babies have the remarkable ability to regenerate after surgery but that adult hearts do not have this capacity. Melbourne Biomedical Precinct researchers are turning stem cells into heart cells and testing a variety of drugs on them to see which ones might trigger this natural regeneration process.

A longer-term project is working towards creating 'designer' stem cells that are engineered to do things that no natural cell can do. These include custom immune cells that seek out and attack the patient's specific cancer, or 'bio-factories' that provide an ongoing source of insulin for a Type 1 diabetic so they don't need regular injections.

Current strengths and opportunities

Leadership

The Melbourne Biomedical Precinct has the largest concentration of stem cell researchers in Australia, helping to drive stem cell science across the country. The University of Melbourne heads Stem Cells Australia, which is an Australian Research Council Special Research Initiative, bringing together the country's top experts in stem cell biology, bioengineering, nanotechnology and clinical research.

Alliance building

Melbourne Biomedical Precinct leaders are in the process of establishing the Melbourne Stem Cell Medicine Alliance to improve coordination and collaboration across the Melbourne Biomedical Precinct and to capitalise on the expertise and capabilities in stem cell science. The Alliance will also help attract funding and support for the people, platforms and projects necessary to drive the future of stem cell medicine.

Clinical trials

Researchers within the Melbourne Biomedical Precinct are advancing towards first-in-human clinical trials using stem cells to repair or replace damaged or diseased cells to restore normal function. More trials could be run in Victoria within the next five years, covering common diseases like Parkinson's, heart disease and macular degeneration, among many others. Hosting clinical trials in Melbourne will advance the science and commercialisation of stem cell therapies and also give Victorians access to the latest treatments for these diseases.

Population matching

There is opportunity to create a biobank of high-quality stem cells matched to the Australian population, called a haplobank, which can be used to generate cells for use in clinical trials. The Melbourne Biomedical Precinct already houses the Bone Marrow Donor Institute (BMDI) Cord Blood Bank, which is a potential source of cells to make high-quality iPS cells that can then be used to generate a wide variety of different cell types for clinical trials. By selecting cords that are 'superdonors', it will be possible to generate iPS cells that could be compatible with a substantial proportion of Australian patients. This will also make Victoria an attractive location for clinical trials.

Future opportunities

Stem cell medicine embedded in health care

Stem cell research cuts across multiple fields, which requires coordination and collaboration between Precinct Partners to extract maximum value from the science. Integrating stem cell science with genomics and industry in the Melbourne Biomedical Precinct and co-locating these within walking distance of hospital and patient facilities will drive clinical trials and first in human studies. This would accelerate the development of more precise and personalised drug treatments based on stem cell models and also give the rapidly growing regenerative medicine industry a home in Melbourne.

Building industry partnerships

Where possible, partnering with industry is an opportunity to develop integrated infrastructure for screening and the collaborative input of the engineers, stem cell biologists and pharmaceutical scientists.

Information sharing and community engagement

State and national policy should address the regulatory, privacy and consent issues involved in stem cell medicine in a legally sound way. This ensures benefits of stem cell medicine translate to patients and are respectful of the rights of individuals and communities.