



Research Platform 03

Cell and biological therapies

Defining cell and biological therapies

In contrast to pharmaceutical small molecule drugs, cell and biological therapies use substances made from living organisms to treat disease. These substances may occur naturally in the body or be made in the laboratory. Some biological therapies stimulate or suppress the immune system to help the body fight cancer, infection and other diseases. Other biological therapies attack specific cancer cells, which may help keep them from growing or kill them. They may also lessen certain side effects caused by some cancer treatments. Types of biological therapy include immunotherapy (such as vaccines, cytokines and some antibodies), gene therapy and some targeted therapies.

There is untapped potential in the Melbourne Biomedical Precinct to develop and commercialise biological therapies that are revolutionising how we treat many diseases including cancer, potentially leading to a multi-billion dollar industry.

Cancer immunotherapy represents the biggest shift in the treatment of cancer for decades and promises to fundamentally change how we think about and treat cancer. It is plausible that within 15 to 20 years many cancers will no longer have the devastating effects they have today. They will become chronic diseases, treatable with minimal impact on a patient's life. This means better outcomes for the patient and indirect benefits to the economy by keeping people healthier for longer.

We are already witnessing the potential benefits of immunotherapy. One type of immunotherapy uses monoclonal antibodies, which are reengineered versions of the antibodies produced by our immune system that are

programmed to hunt down 'foreign invaders' such as bacteria and viruses. When the antibodies are injected, they seek out and attach to the surface of the tumours, either blocking their ability to grow or attracting other immune cells to come along and kill them. Monoclonal antibodies can also be engineered to perform other tasks, such as delivering a drug to a very specific location in the body, which prevents it from affecting unwanted cells. Antibody treatments are some of the top selling pharmaceutical biological therapies providing positive effects for adult patients in hard-to-treat cancers like melanoma and lung cancer, which combined kill over 10,000 Australians each year.

Another type of immunotherapy currently in clinical trials is Chimeric Antigen Receptor (CAR) T-cell therapy. This is showing positive results on children with acute lymphoblastic leukaemia (ALL), effectively eliminating the cancer from these vulnerable patients and returning them to full health. ALL is the most common form of childhood cancer, with around 180 Australian children diagnosed each year. Existing chemotherapy and radiotherapy treatments for ALL are costly and have serious side effects and around 20 per cent of children don't respond

to treatments at all. The first CAR T-cell vaccines have recently been approved for use in the United States and have the potential to revolutionise the treatment of many intractable cancers besides leukaemia, such as lung, pancreatic and brain cancers.

There are other kinds of cell and biological therapies that are also generating a great deal of excitement. Stem cell applications, such as regenerative medicine, which uses the body's own cells to heal itself, offer significant potential. One application being investigated within the Melbourne Biomedical Precinct is using 3D bio-printers that can use a patient's own cells to 'print' replacement bone to repair an injury or damage from disease. Another form of biological therapy with a longer lead-in time is engineering new stem cells to perform a specific function, such as creating cells that produce insulin and implanting them in a patient with type 1 diabetes.

Current strengths and opportunities

Collaboration

Pharmaceutical companies globally have already demonstrated a significant interest in cell and biological therapies. More than 40 per cent of treatments approved by the US Food and Drug Administration in recent years have been for biological therapies rather than the traditional small molecule drugs. Many international pharmaceutical companies are currently seeking to collaborate with innovative researchers, particularly using immunotherapy to combat cancer.

Market expertise

The Melbourne Biomedical Precinct is well recognised for its work, receiving a majority of Australia's research grant funding in cell and biological therapies. Australia's largest biotechnology company and biologicals specialist, CSL, is based in the Melbourne Biomedical Precinct. Precinct Partners have access to CSL's depth of expertise in research and development and regulatory processes for new therapies and skills required to increase speed to market. Cell Therapies Ltd operates the largest and highest grade biologicals manufacturing facilities in Australia and is based at the VCCC. Precinct Partners have access to facilities and resources to take breakthroughs in the lab to first-in-human trials and through the development and commercialisation process.

Information and clinical trials

The Melbourne Biomedical Precinct is ideally situated to handle clinical trials, given the ethnic and genetic diversity of the Victorian population, along with the presence of top-quality researchers, clinicians and hospitals.

Future opportunities

New biological therapies

A significant increase in the development of new biological therapies is expected over the next decade. Attracting industry investment into the Melbourne Biomedical Precinct is critical to support this development, by building on current capacity and expertise for clinical trials. While the private sector has expressed great interest in new biological therapies, pharmaceutical companies typically only invest once a new treatment has been developed beyond the basic research stage and efficacy and safety has been demonstrated in early-stage clinical trials. Precinct Partners must therefore work together to take new innovations from the lab into first-in-human trials and larger multi-site trials will also be a key priority.

Manufacturing growth

Victoria has the expertise to produce biological therapies, but could also increase its manufacturing capacity in this area. While such facilities may not be able to be accommodated within the Melbourne Biomedical Precinct itself, other areas across Melbourne are well-placed to support this development, including the south east corridor, CSL's soon-to-recently opened expanded Broadmeadows facilities and Arden in the longer term.

Commercial expertise

Access to experts and staff trained in commercialisation would enable researchers to advance their work rapidly to the point where it can attract the external funding necessary to drive it through the commercialisation process.