

Building Capacity for Human Cellular Diagnosis and Therapy

Manipulation and transplantation of isolated cells, rather than whole organs or tissues, is an emerging area of diagnostic and therapeutic medicine. Leading-edge cell therapy research is an area of strength and growth in Victoria due to substantial previous investment. The aim of this program is to further enhance Victoria's capability in cellular therapy by developing a framework for the development and early application of emerging cell therapies and associated diagnostics.

There are three nodes in the current development:

NODE 1: Development of core technology for human cellular diagnosis

Diagnostics that characterise disease, assess disease risk and measure therapeutic response will be a key component of personalised medicine. To build capacity for human cellular diagnosis, we are building a facility for flow cytometric isolation and analysis of human cells at Melbourne Health that will complement related facilities at the Peter MacCallum Cancer Centre and the Ludwig Institute/Austin Health. This is core technology for current and future cell therapies.

By allowing cells to be isolated and analysed before and after therapy, this facility will:

- improve diagnostic monitoring procedures e.g. the FACS Aria will allow the assessment of acute rejection post-islet and post-liver cell transplantation (Nodes 2 and 3)
- enable researchers to isolate and analyse specific cells to gain new insights into the defects that underlie many diseases
- allow cells to be manipulated in the laboratory for re-infusion or re-implantation to repair or replace abnormal cells or tissues. This will allow researchers to bring new cell therapies to the clinic and to monitor the effects of these therapies.

NODE 2: To deliver a pilot trial of human islet allografting for patients with unstable type 1 diabetes

We will build capacity for clinical cellular therapies by demonstrating the effectiveness of adopting non-haemopoietic cellular therapies into clinical practice to treat a common disease, type 1 diabetes. In a collaborative program initiated by St Vincent's Health, St Vincent's Institute and Austin Health, human islets isolated in the Centre for Blood Cell Therapies at Peter MacCallum Cancer Centre will be transplanted to treat people with unstable type 1 diabetes. Our specific project objectives are to establish a clinical transplant program based on the Edmonton Protocol that is known to deliver a high degree of patient safety and success, and then to progress the procedure by using what we learn about human islet function in the laboratory to improve the survival of transplanted islets and reduce the need for immunosuppression. The ability to deliver high-quality clinical programs in non-haemopoietic cell transplantation will be of great benefit to future cell therapy trials including xenotransplantation, prevention of primary graft non-function, and tissue engineering. The program, called the Tom Mandel Islet Transplant Program after an Australian pioneer in the field of islet transplantation, will be officially launched in June 2005.

NODE 3: To deliver a clinical liver cell transplant program to treat liver failure or dysfunction in paediatric patients

The new Cell Therapeutic Unit at the Royal Children's Hospital /Murdoch Children's Research Institute (RCH/MCRI) has recently been completed. The facility is designed to accommodate laboratories for the closed system manipulation of bone marrow and cord blood samples, a GMP-certified cellular therapies unit for the *ex vivo* expansion of stem cells, a tumour vaccine production laboratory and laboratories for MCRI translational research activities with embryonic and adult stem cells.

The MCRI/RCH has recently performed Australia's first liver cell transplant and several patients are currently being assessed to be wait-listed for a cell transplant.

NODES 1, 2 and 3: To develop new cell therapies at the pre-clinical level

Major improvements in the supply of insulin-producing and liver cells, viability of isolated islet cells and immunosuppression are required before cell therapy approaches can become a broadly applicable approach that will reduce the burden of disease in the community. Once the clinical transplant programs are established, our goal will be focused on developing research programs that will improve the efficiency of the isolation procedures, protect human cells and allow the development of new clinical strategies that will improve the transplantation procedure.

NODES 1, 2 and 3: To develop a Victorian Cell Therapy Network

Collaborations between Victorian organizations that are involved in developing or implementing novel cellular therapies will be strengthened and expanded by establishing a Victorian Cell Therapy Network. By promoting the sharing of assets, resources, research talent and know-how, the network will help create platforms for trialling new cellular diagnoses and therapies in the future. The VCTN will be launched at a workshop later in 2005.

Further Information

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