Stem Cell Network | Réseau de Cellules Souches

Powering Regenerative Medicine
Propulsons la médecine régénératrice

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Stem Cell Network's Horizon 2022 Award Winners — Fighting Disease, Saving Lives, and Powering the Growth of Regenerative Medicine in Canada

Imagine a future where therapies exist to treat rare and chronic diseases and life-altering illnesses such as multiple sclerosis, cardiovascular disease, type 1 diabetes, Parkinson's disease and muscular degeneration. Science is getting close – and Canada is helping pave the way.

Traditionally powered by stem cells, the field of regenerative medicine focuses on replacing, repairing, or regenerating human cells, tissues, and organs. It is considered by many to be the next frontier of medicine. Known widely as the "building blocks" of the body, stem cells have tremendous potential to treat a plethora of diseases, injuries, and life-altering illnesses, and Canada has been a global leader in this field for over 60 years.

Canada's Stem Cell Network (SCN) has been leading the way in building national capacity in stem cell and regenerative medicine research for over 20 years. On May 12, 2022, and with support from the Government of Canada, SCN announced $19.5M in funding for 32 regenerative medicine research projects and clinical trials involving more than 400 researchers, clinicians, and trainees from across the country. Impressively, it is the single largest investment in regenerative medicine research in the Network's history.

The largest of the projects funded by SCN are part of a new multi-year program, **the Horizon awards**. Valued at $3M per award, they are the largest award SCN has offered in the past decade and focus on conducting innovative research and developing transformative technology solutions for tackling regenerative medicine challenges that will result in both health and economic benefits in the coming decade. Horizon awards also aim to seed research and build made-in-Canada intellectual property that will yield translational or commercialization activity.

Three multi-disciplinary national teams, led by **Drs. Sara Vasconcelos**, **Guy Sauvageau**, and **James Shapiro**, have been granted Horizon awards. Their research will focus on advancing cardiac regeneration, next-generation therapies for treating blood cancers, and scaling up and biomanufacturing personalized therapies for type 1 diabetes.

Dr. Sara Vasconcelos — Recycling Fat Blood Vessels to Heal Hearts

Cell transplantation has the potential to regenerate organs after damage caused by various diseases and acute health events, such as heart attacks. However, for cells to survive transplantation and effectively regenerate organs, the cells need immediate access to oxygen and nutrients from blood vessels. Many past attempts at making new blood vessels for organ regeneration have failed, a roadblock that is the focus of Dr. Sara Vasconcelos, a Scientist at the University Health Network in Toronto.

Motivated by the need to generate alternative therapeutic avenues to treat cardiovascular diseases, Dr. Vasconcelos and her team are already making significant strides in addressing this major obstacle for cell-replacement therapies. Heart disease affects 1.3 million Canadians, and the Heart and Stroke Foundation reports it is the costliest disease in Canada, totaling $21.2B in direct (medical) and indirect (lost earnings) costs. This is why the work of Dr. Vasconcelos and her team is so important.

For the first time they have shown an effective means to support cell survival and improved organ function in small animal models, like rats, by recycling blood vessels from fat to support the survival of transplanted cells.

The next step in Dr. Vasconcelos' research is to advance this work using a large animal model and generate the necessary efficacy and safety data to eventually move this therapy into clinical trials. If successful, this research will be a game changer for millions of people.

Dr. Guy Sauvageau — Engineering Blood Stem Cells for Tomorrow's Medicine

The world of stem cell engineering provides us with opportunities for solving some of the most complex medical problems. It's the focus of Dr. Guy Sauvageau's work at the Université de Montréal.

Dr. Sauvageau is the principal investigator at the Institute for Research in Immunology and Cancer (IRIC), where he focuses on using stem cells to produce innovative treatments for blood cancers. Such cancers impact the lives of over 155,000 Canadians annually, and cost an average of $156,000 per patient in the first year following diagnosis, with upwards of $800,000 of costs to the health care system over a three-year period.

Modern cancer therapies increasingly rely on antibody-based drugs or genetically engineered immune cells that are programmed to recognize specific proteins on the surface of cancerous cells while not attacking their healthy counterparts. The trouble is, for many aggressive blood cancers, the leukemic and healthy blood cells express very similar proteins on their surface.

Dr. Sauvageau's team is working to identify proteins with robust expression on leukemia cells and engineer blood stem cells grafts as a next-generation therapy for treating aggressive blood cancers. The goal of this project is to better target leukemia cells while sparing the normal blood system regenerated from engineered stem cell transplants. In addition, Dr. Sauvageau's team is looking to establish an improved delivery method for genetically engineered anti-leukemia immune cells into leukemia patients by completely modifying blood stem cells which would allow for the regeneration of engineered immune cells for life. Overall, this research has the capability to vastly improve therapeutic prospects for leukemia patients.

Dr. James Shapiro — Patient-derived Stem Cells (self-islets) as a Potential Cure for Diabetes

Diabetes is one of the most common conditions, affecting more than 300,000 Canadians. The Public Health Agency of Canada reports that over the past 10 years new cases of diabetes cost Canada's health care system $15.36B. Diabetes is caused by the lack of insulin, a hormone produced by the islet beta cells in the pancreas, responsible for regulating blood sugar. Efforts to transplant islet beta cells have been successful in regulating blood-sugar levels in some patients with type 1 diabetes but doing so means patients have to deal with lifelong anti-rejection drugs, and a shortage of donor organs hampers a wider rollout.

For patients facing a lifelong journey of blood sugar monitoring, insulin injections, and careful meal planning, what if there was a treatment and eventually a cure? Dr. James Shapiro, Professor of Surgery at the University of Alberta and Canada Research Chair in Transplantation Surgery and Regenerative Medicine, is looking to cutting-edge stem cell research for the answer.

Dr. Shapiro is using his 21 years of experience in islet transplantation (including his ground-breaking Edmonton Protocol for islet cell transplants) to use a patient's own cells as the solution for their type 1 diabetes. His team's strategy is to use beta cells grown from a patient's stem cells to replace the damaged beta cells in people with various forms of diabetes, including type 1, type 2, and surgical diabetes caused by the partial removal of the pancreas. Using a patient's own stem cells to make the "self-islets" will help the transplanted cells to be accepted by the patient's immune system and remove the need for anti-rejection drugs.

The team will also address a pressing Canadian biomanufacturing gap, and work to scale-up the production of sufficient self-islets for transplantation into patients, and the self-islets will then be evaluated in a clinical trial. Ultimately, Dr. Shapiro and his team hope to develop a scalable solution to advance the therapies for millions of people living with diabetes across the globe.

The Way Forward

Not only are these three investigators and their multi-disciplinary research teams providing genuine hope for Canadians of better health and treatments, but they are also building a solid foundation for growth of Canada's biomanufacturing and life sciences sector. It is projected that the regenerative medicine sector will provide $5B in potential growth for the Canadian economy and the promise of thousands of jobs in the coming years.

With stable and predictable support from the Government of Canada and our partners along with SCN's steadfast commitment to research excellence and the translation of innovations, we will unlock the immense potential of regenerative medicine. SCN's world-leading researchers are now poised to take on the complex challenges that arise from chronic and rare disease and develop the life-changing and life-saving treatments needed by patients while accelerating Canadian innovations to the global marketplace.