STEM CELL NETWORK

Tomorrow's health is here.

InFocus

REGENERATIVE MEDICINE INNOVATIONS FOR HEALTH

Stem cells and regenerative medicine offer a myriad of opportunity to address chronic illness in Canada to reduce the burden of health care and increase productivity. Since 2001, the Stem Cell Network has played a pivotal role in advancing research to bring new products and therapies to market.

With engagement from more than 170 research groups and 115 members and partners, the Stem Cell Network has positioned Canadian stem cell discoveries and innovations at the forefront of a competitive global landscape.

STEM CELL NETWORK AT A GLANCE 2001-2019

$118 Million
direct investment in Research, Training & Outreach

$116 Million
in Research Partnerships

200 Translational research projects supported across
170 Research teams

879 Patent applications
115 Patents issued
94 Licenses

3,067
Trainees & HQP supported

19 Clinical trials
21 RM biotechs

In the Pipeline

Neurobioink

3D bioprinting can generate engineered tissue constructs by delivering cells in a supportive bioink that promotes their growth and differentiation, forming appropriate physiologically relevant structures. Human pluripotent stem cells and their derivatives tend to be more sensitive to printing conditions compared to mature cells, making them more difficult to bioprint while ensuring good viability and differentiation. Stephanie Willerth at the University of Victoria is a leader in this field, and has developed a novel neurobioink capable of printing hiPSC-derived neural cells that maintains a high level of cell viability. Despite a demonstrated demand, there are currently no similar bioinks on the market or emerging equivalents. The advantages of the Willerth lab's neurobioink include:

- Compatible with multiple bioprinting systems
- Generates stable constructs for over a month
- Less labour intensive, fewer productions steps
- Increased reproducibility
- Promotes neuronal differentiation
- High-throughput compatible

STEPHANIE WILLERTH,
University of Victoria >

< BERNARD THÉBAUD,
Ottawa Hospital

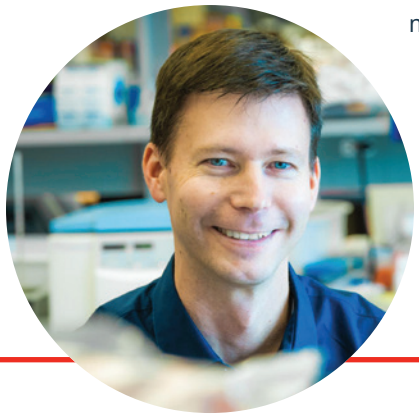
Engineered Gene Rescue

Monogenic lung diseases (those caused by a mutation in a single gene) include a variety of disorders, such as cystic fibrosis and surfactant protein deficiencies, that cause chronic lung disease, are often fatal, and have few targeted therapies. Bernard Thébaud at the Ottawa Hospital has engineered a gene therapy using adeno-associated virus (AAV) that could help cure monogenic diseases. Preclinical studies have shown that the AAVenger (AAV Engineered GEne Rescue) platform dramatically improves gene expression, lung function, and survival in mouse models surfactant protein B deficiency, a lethal newborn lung disease. Given the unprecedented lung transducing efficiency of this vector, future development includes expanding the AAV platform to extend the therapeutic applications to a variety of monogenic lung diseases. This rationally-engineered AAV vector has a number of beneficial attributes:

- Rapid therapeutic effect
- Reduced cost of vector production
- Improved lung gene transfer capabilities
- Preferential targeting of cells that express the defective protein
- Potential application for large gene replacement and gene correction

Bioprinted Pancreatic Tissue

A tissue-based therapy for type 1 diabetes has a potential market of nearly $10B in North America. Leading diabetes researcher, Timothy Kieffer at the University of British Columbia has partnered with Vancouver-based Aspect Biosystems to develop a proprietary bioprinting technology to create implantable tissue patches that mimic pancreatic islet function. The technology incorporates beta cells derived from human pluripotent stem cells into 3D printed tissues that are retrievable but also amenable to long-term viability and sustained immune evasion. In vitro optimization of bioprinted tissue implant design is being complemented with validation in animal models to yield a robust pre-clinical data package leading to first-in-human studies. The implementation of the bioprinted pancreatic tissue for patients living with type 1 diabetes will most likely occur via a centralized manufacturing facility that operates to GMP standards and through licensing of Aspect's bioprinting technology. As a potential therapy for type 1 diabetes, the bioprinted tissue patch could transform the lives of individuals with this disease as well as their families.



< TIMOTHY KIEFFER,
University of British Columbia

Companies to Watch: Emerging Leaders

Aspect Biosystems is a privately held biotechnology company operating at the leading edge of 3D bioprinting and tissue engineering. The company is built on over 10 years of research and development and formed through a collaboration between world-class research groups in Engineering and Medicine at the University of British Columbia. Led by co-founder and CEO Tamer Mohamed, Aspect's proprietary Lab-on-a-Printer™ platform technology is enabling advances in understanding fundamental biology, disease research, development of novel therapeutics, and regenerative medicine. Aspect is focused on strategically partnering with pharmaceutical and biotechnology companies, as well as academic researchers, to create physiologically and commercially relevant tissues. These tissues are used to advance and accelerate drug discovery and development, and enable the creation of cutting-edge tissue therapies of the future. **aspectbiosystems.com**

Montreal-based ExCellThera is at the forefront of global efforts to increase the quality and quantity of healthy blood stem cells available to treat people with blood malignancies. This privately-held company is led by Co-founder and CEO, Guy Sauvageau, whose team discovered the UM171 molecule, which can substantially increase the number of stem and immune cells for therapeutic use. Using this molecule in combination with ExCellThera's optimized culture system means that cells can be ready in as little as seven days – three times faster than the competition – which can be critical for patients. Moreover, the results from early phase clinical trials show that the technology significantly reduces the complications of transplantation, providing better recovery and fewer days spent in hospital. With the support of the Stem Cell Network, ExCellThera has emerged as a Canadian commercialization success and is now expanding its reach into the United States and Europe. **excellthera.com**

Companies to Watch: Start-ups

MORPHOCELL technologies™

Montreal has emerged as a hotbed for biotech startups in recent years and one of the newest entries is Morphocell Technologies, a company founded by Massimiliano Paganelli and Claudia Raggi in 2018 to develop and commercialize stem cell therapies and engineered tissues aimed at treating liver diseases. Stem Cell Network grants enabled Paganelli and his team to develop stem cell-derived tiny liver organoids, which are encapsulated in a special biomaterial to form a tissue that performs like a human liver. When transplanted into a patient, this tissue, ReLiver™, replaces the key vital functions of the diseased liver, while accelerating its regeneration and healing. This technology has the potential to prevent up to 80% of liver transplants for acute liver failure. The technology has shown immense promise in animal models to restore liver function in case of severe acute liver failure, significantly improving survival and preventing/treating hepatic encephalopathy. The company is seeking to initiate human clinical trials within the next two years. In addition, Morphocell is developing ReLiver™ to treat acute-on-chronic and chronic liver failure. **morphocell.com**

Satellos bioscience

Leading stem cell researcher and SCN Scientific Director Michael Rudnicki has a bold vision to more effectively regenerate muscle tissue, which formed the impetus for the creation of Satellos. Effective muscle repair requires resident stem cells to successfully balance the production of new muscle tissue with replenishment of the stem cell pool. However, this process can become imbalanced by injury, chronic illness, disease or aging, effectively impairing muscle regeneration and function. Satellos' novel platform uses a pharmacologic approach to restore this balancing act for greater muscle repair and healing. This Canadian technology has the potential to enhance regeneration for the treatment of a range of neuromuscular diseases. The company has invented novel drug candidates, established a strong management team and is seeking Series A financing to continue development of its unique approach to tissue repair and regeneration. **satellos.com**

StemAxon™

StemAxon CEO and Co-founder Gilbert Bernier is another Stem Cell Network investigator who has launched a promising biotech firm in Quebec. StemAxon is pursuing breakthroughs in both neurodegenerative diseases such as Alzheimer's and retinal diseases, and could be a world leader when it comes to using neural cell transplantation to treat both conditions. The company provides an innovative platform to test compounds against Alzheimer's directly, thereby speeding the discovery of potential new treatments. Through StemAxon, and with the support of SCN, Dr. Bernier is also exploring how neural cell transplantation can treat macular degeneration and other retinal conditions that lead to blindness. Having discovered how to grow stem cells into the cone photoreceptors needed for functional eyesight, Dr. Bernier and the StemAxon team are now in the process of moving their discoveries to the clinic. **stemaxon.com**