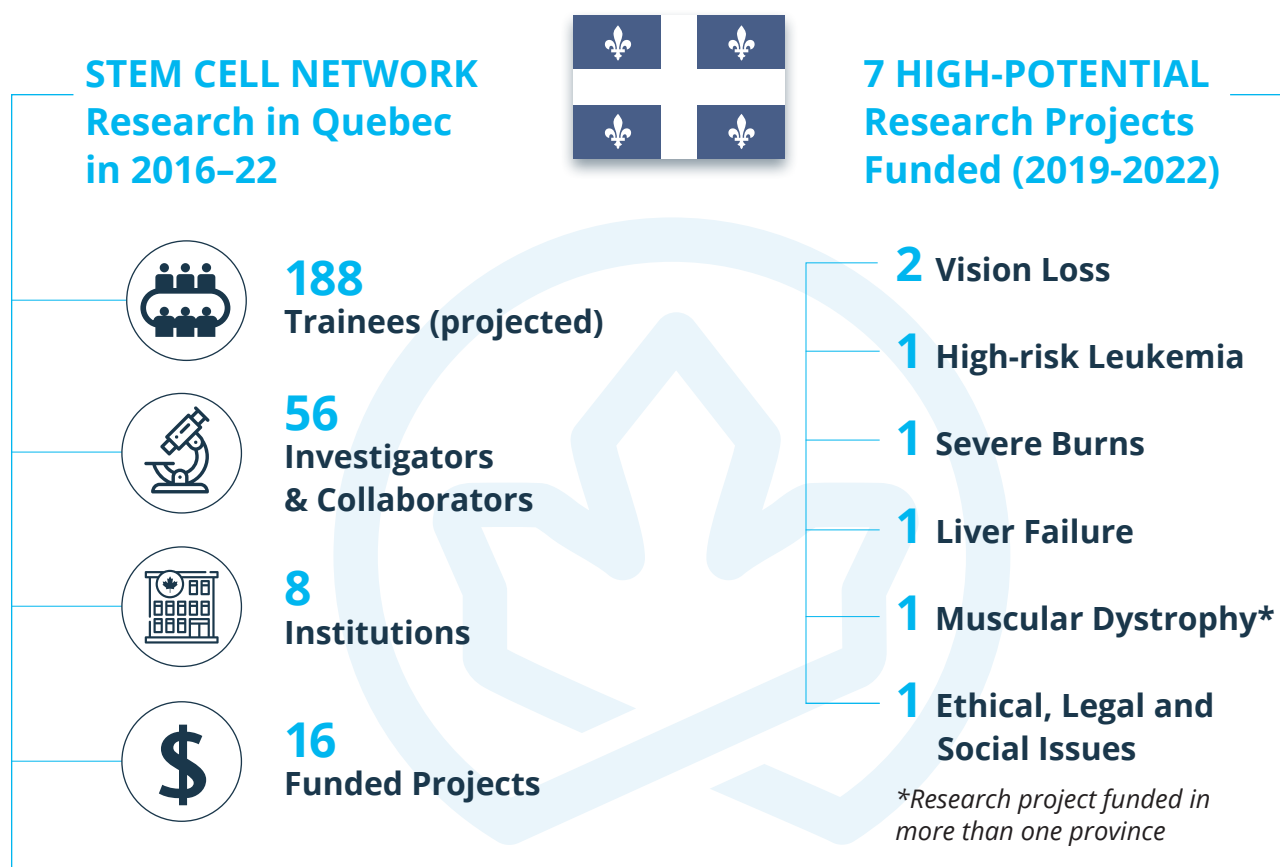




# Infocus

## STEM CELL INNOVATION IN QUEBEC

Scientists in Quebec have been at the cutting edge of stem cell research for more than 20 years. There is immense potential in the work of investigators across the province, many of whom have benefitted from Stem Cell Network (SCN) support. Since 2001, SCN has funded 59 Quebec-based projects valued at \$13.7M, including over \$6.4M from 2016–2022. Stem cell discoveries made in Quebec have global implications for the treatment of blood, eye and liver diseases, and for patients recovering from severe burns and skin injuries.



## Advancing to the Clinic



**DR. VÉRONIQUE MOULIN,**  
Université Laval

Extensive burn wounds can take a long time to close and often do not heal completely, resulting in increased mortality and/or long-term complications. While significant progress has been made over the past 25 years in regenerative medicine, the permanent coverage of extensive burns continues to be a challenge.

Dr. Véronique Moulin at Université Laval is advancing a clinical trial based on her team's development of advanced tissue-engineered skin substitutes that can be produced from only a small skin biopsy and could permanently cover an entire burn area. The new skin, referred to as self-assembled skin substitute (SASS), allows the replacement of both layers of skin (dermis and epidermis) in a single surgical procedure. So far, 20 patients in Quebec and Alberta have been treated with SASS, with positive results. With the support of the Stem Cell Network, this clinical trial will be expanded to include patients from five new centres in Alberta, BC, Manitoba and Ontario over the next two years. It is expected that the SASS treatment will result in important health and social benefits. If successful, this treatment will improve upon the current standard of care – most notably, by decreasing pain for patients and reducing the need for further surgery, while improving the quality of post-burn scars.

**DYK?**

**PEOPLE WITH SEVERE BURNS SPEND AN AVERAGE OF 27 DAYS IN HOSPITAL. MEN ARE TWICE AS LIKELY AS WOMEN TO BE SEVERELY BURNED, BUT CHILDREN, WHOSE SKIN IS THINNER, BURN FOUR TIMES MORE QUICKLY AND DEEPLY THAN AN ADULT. THEIR RAPID PHYSICAL GROWTH MEANS CHILDREN ALSO SCAR MORE EASILY.**

## Clinical Trials in Quebec

Since 2016, the Stem Cell Network has supported five cell therapy clinical trials in Quebec valued at nearly \$2.5 million. These trials are advancing leading research in the following areas:

- Blood stem cell expansion technology for patients with high-risk blood disorders such as leukemia and multiple myeloma. Three phase I-II clinical trials have been supported at the primary treatment centre, the Maisonneuve-Rosemont Hospital in Montreal.
- Self-assembled skin substitute for patients with severe burns. One phase I-II trial is being supported at multiple centres across Canada.
- Corneal autografts for patients with vision impairments caused by limbal stem cell deficiency. One phase I-II trial is being supported at the Centre universitaire d'Ophtalmologie CHU de Québec in Quebec City.

# Disrupting the Field

In his recently established lab at the Université de Sherbrooke, former SCN trainee Dr. Florian Bentzinger is turning over new stones in the search for a treatment for muscular dystrophy, a group of rare and incurable diseases that often affects young children. Muscular dystrophies affect the stem cells responsible for natural regeneration of muscle tissue, which over time can result in weakness, reduced motor function and early mortality.

Bentzinger and his team have identified both new and repurposed drugs that can stimulate muscle stem cell function, improve motor function and reduce disease progression. Using a drug-based approach has several benefits including removing the need to culture cells in a laboratory or transplant them using potentially invasive surgeries. With research support from SCN, this team is aiming to bring the most promising of these drugs towards clinical trials. It's a first-in-class and highly disruptive discovery with the potential to have a dramatic impact in the field of regenerative medicine.



**DR. FLORIAN BENTZINGER,**  
Université de Sherbrooke

**THERE ARE MORE THAN 160 FORMS OF MUSCULAR DYSTROPHY, ALL OF WHICH ARE CAUSED BY A GENETIC MUTATION AFFECTING THE CELLS THAT BUILD MUSCLE. THE MOST COMMON FORM OF MUSCULAR DYSTROPHY IS DUCHENNE, WHICH TYPICALLY AFFECTS BOYS BEGINNING AT ABOUT AGE FOUR.**

**DYK?**



Stem cells are our bodies' building blocks and our master healers. Because stem cells can repair tissues and organs, they are powering regenerative medicine, offering new ways to improve quality of life for patients and reduce the health care burden.

There is enormous potential for stem cell therapies to treat chronic diseases and debilitating illnesses such as:

- |                      |                    |                    |
|----------------------|--------------------|--------------------|
| Alzheimer's          | Blood disorders    | Brain injury       |
| Crohn's disease      | Diabetes           | Heart disease      |
| Liver disease        | Muscular dystrophy | Multiple sclerosis |
| Respiratory diseases | Septic shock       | Vision impairments |

The Stem Cell Network is Canada's stem cell research organization. It is committed to working with researchers from coast to coast to get new therapies and medicines to market and to those who need them most.

# Commercialization Success



Getting new stem cell therapies to patients is the ultimate goal for Quebec researchers. To do so, some researchers have launched biotech companies that aim to commercialize the promising stem cell-based technologies they've engineered.

One of the newest entries is Morphocell Technologies, a company founded in 2018 by Drs. Massimiliano Paganelli and Claudia Raggi to advance stem cell therapies and engineered tissues aimed at treating liver diseases. Stem Cell Network grants enabled this team to develop 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 regeneration and healing. This technology has the potential to prevent up to 80% of liver transplants for acute liver failure. In addition, Morphocell is developing ReLiver™ to treat acute-on-chronic and chronic liver failure. The company recently added to its talent pool with the acquisition of Dr. Jennifer Moody as Chief Operating Officer, who will use her stem cell commercialization and business operations expertise to help guide Morphocell into clinical trials within the next two years.

**DYK?**

THERE ARE OVER 100 LIVER DISEASES, AFFECTING 1 IN 4 CANADIANS. THE CAUSES OF LIVER DISEASE IS VARIED AND INCLUDE VIRUSES, TOXINS, GENETICS, ALCOHOL AND UNKNOWN CAUSES.

## SCN's 2016–22 Quebec Partners

AccellAB	Institut de pharmacologie de Sherbrooke
Canadian Cancer Society Research Institute	IRICoR
Centre de recherche du CHU de Québec – Université Laval	Lady Davis Institute, Jewish General Hospital
Centre de recherche du CHU de Sherbrooke – Université de Sherbrooke	LOEX
Centre of Genomics and Policy, McGill University	Morphocell Technologies
CHU Sainte-Justine Foundation	Quebec Ministère de l'Économie et Innovation
ExCellThera Inc.	Quebec Network for Research on Aging
Faculté de médecine et des sciences de la santé (FMSS) de l'Université de Sherbrooke	Richard and Edith Strauss Foundation
Felicia & Arnold Aaron Foundation	SOVAR
Hôpital Charles-Le Moyne	StemAxon
Hôpital Maisonneuve-Rosemont	ThéCell Network
	Univalor
	Zamboni Chem Solution Inc.

SCN's partners provide important support for Quebec-based stem cell research. Investigators in Quebec have leveraged over \$13M in partner contributions on the \$5.5M provided by SCN. This investment will see new therapies move more rapidly towards the clinic, increasing access to those who are most in need.