Stem Cell Network
Réseau de cellules souches

InFocus

2018

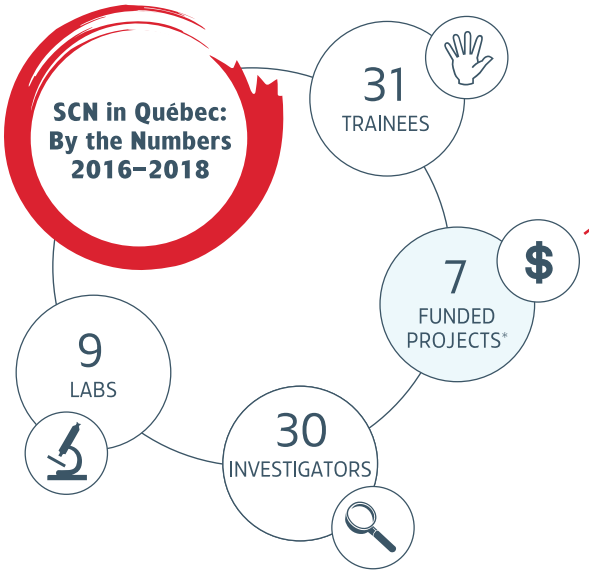
In Quebec

MAKING AN IMPACT

STEM CELL INNOVATION

Scientists in Quebec have been at the cutting-edge of stem cell research for nearly 20 years. Recognizing the potential of investigators working and collaborating in the province, the Stem Cell Network (SCN) has supported 47 projects with $9.3M in funding since its inception, including over $2M in 2016–18 alone. Stem cell discoveries made in Quebec have global implications for the treatment of eye and liver diseases, and for neurodegenerative diseases such as Alzheimer's. It was also in Quebec where a major advance in blood stem cell expansion took place, boosting quantities of hematopoietic stem cells vital for treating blood diseases, including blood cancers such as leukemia.

Stem Cell Network Research in Quebec 2016–18



$2.0M

7 HIGH-POTENTIAL Research Projects Funded + $5.0M Partner Support

2 CORD BLOOD – STEM CELL EXPANSION†

1 LIVER FAILURE

1 MUSCLE STEM CELLS

3 OTHER**

$9.3M invested (2001–2018)

***Cornea limbal stem cell deficiency, degenerative diseases of musculoskeletal system, gene therapy and immunotherapy†*

†Research project active and funded in more than one province

**In 2016–2018, SCN funded 25 research projects and 6 clinical trials nationwide.*

DYK?

38% of principal investigators (PIs) and co-investigators for Quebec projects are women.

Disrupting the Field: Massimiliano Paganelli

One in ten people in Canada has a liver disease, and many of these diseases progress to liver failure. Currently available treatments for liver failure are inadequate and the death rate from liver-related illnesses is on the rise. At the Centre hospitalier universitaire Sainte-Justine in Montreal, Dr. Massimiliano Paganelli is seeking better ways to treat liver failure using a novel stem cell therapy. His project, supported in part by SCN, is geared towards developing a stem cell-based product to replace liver functions in both children and adults with liver failure. Dr. Paganelli and his team use stem cells to create thousands of mini-livers called organoids, which work together within a special biomaterial to form a tissue that performs like a human liver. This "encapsulated liver tissue" is ten to a thousand times more effective than any other system ever developed. It has the potential to be transplanted through minimally invasive surgery into patients with liver failure, and to restore liver functions without the risk of rejection or the need for immuno-suppression. Dr. Paganelli is a dedicated pediatric clinician whose research has the potential to benefit people of all ages.

Dr. Massimiliano Paganelli,
CHU Sainte-Justine

Disrupting the Field: Lucie Germain

SCN has been funding Dr. Lucie Germain since 2011. In 2016, she was awarded $90,000 to support her work creating new treatments for those with corneal conditions. This work has value for the nearly one million people in Canada affected by vision loss, which is estimated to cost more than $15.8B each year (2007 figures). Dr. Germain is a Canada Research Chair in Stem Cells and Tissue Engineering based at Université Laval, and a leading expert in reconstructing damaged organs — especially skin, corneas, blood vessels and valves — with stem cell technologies. Her work has led to clinical trials to test new ways to repair corneal stem cell deficiency (eye) and to treat severe burns. Dr. Germain and her team are also looking at innovative stem cell treatments for skin conditions that produce blisters and other wounds in patients, or that cause disfiguration.

Dr. Lucie Germain,
Université Laval

From Bench to Bedside: Commercialization Successes

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.

EXCELLTHERA

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 leukemia and lymphoma. The biotech start-up is led by Dr. Guy Sauvageau, with support from investigators Drs. Anne Marinier and Sandra Cohen. This team discovered the UM171 molecule, which can increase the number of useable blood stem cells by a hundredfold. Using this molecule in combination with ExCellThera's small bioreactor 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 company's cord blood expansion product already costs a fraction of what other treatments do, positioning ExCellThera as a commercialization success even in these early days. With the networking and financial help of SCN, ExCellThera has collaborated with multiple partners to move promising treatments towards clinical trial.

StemAxon™

StemAxon CEO and Co-founder Dr. 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.

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

- *Parkinson's disease*
- *Crohn's disease*
- *Muscular dystrophy*
- *Retinal degeneration*
- *Kidney disease*
- *Septic shock*
- *Multiple sclerosis*
- *Leukemia and other cancers*
- *Respiratory diseases*
- *Brain injury*
- *Diabetes*
- *Heart disease*
- *ALS*

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.

Quebec: A stem cell policy hub

Dr. Erika Kleiderman, McGill University

Policy has always played an important role among SCN stakeholders because the discovery and commercialization of new stem cell therapies rely on public policy keeping pace with science (and vice versa). Training the next generation of policy experts is a priority for SCN, and Erika Kleiderman, a Quebec lawyer based at McGill University's Centre of Genomics and Policy, is a shining example of the talent that SCN is supporting.

> *"Stem cell [research] is a fascinating field and with every new discovery and technology it brings an abundance of debates around the ethical nature of research and its implications."*

Ms. Kleiderman first learned about stem cells in high school and was excited to discover a field that held much promise for treating disease. Her interest reignited in university with the emergence of gene editing. Today, she finds herself in the midst of research on ethical, legal and social implications surrounding regenerative medicine, gene editing and other emerging technologies related to stem cells. She is mentored by one of Canada's most noted policy experts, Dr. Bartha Maria Knoppers. With the support and guidance of Dr. Knoppers, Ms. Kleiderman is providing important legal contributions on topics such as genetic data access and biobanking.

DYK?

Cell manufacturing is important for the controlled production of innovative cell therapy products to treat disease. The Centre d'excellence en thérapie cellulaire (CETC) based in Montreal, has the biggest academic cell therapy manufacturing centre in Canada.

SCN's 2016–18 Quebec-based Partners

- Canadian Cancer Society Research Institute
- Centre de recherche du CHU de Québec – Université Laval
- Centre de recherche en organogénèse expérimentale de l'Université Laval/LOEX
- Centre of Genomics and Policy, McGill University
- CHU Sainte-Justine Foundation
- ExCellThera
- Fonds de partenariat pour un Québec innovant et en santé
- Hôpital Charles-Le Moyne
- Hôpital Maisonneuve-Rosemont
- IRICoR (Institute for Research in Immunology and Cancer)
- Lady Davis Institute, Jewish General Hospital
- Quebec Network for Research on Aging
- Richard and Edith Strauss Foundation
- Univalor
- Zamboni Chem Solution Inc.

SCN has 28 partners providing important support for Quebec-based stem cell research in 2016–18. Partnerships are critical for ensuring high-potential research is funded appropriately. Investigators in Quebec have leveraged over $5M in partner contributions on the $2M provided by SCN. This investment will see new therapies move more rapidly towards the clinic, increasing access to those who are most in need.