



Stem Cell Network
Réseau de **cellules souches**

Catalyzing tomorrow's breakthroughs
Catalyseur de l'innovation

Stem Cell Network
Pre-Budget Submission to the
House of Commons Standing Committee on Finance

Dr. Michael Rudnicki, O.C.
Scientific Director & CEO
Cate Murray, Executive Director & COO
Stem Cell Network

The Stem Cell Network (SCN) would like to thank the House of Commons Standing Committee on Finance for the opportunity to provide input into the budget process and address the theme: *“Economic Growth: Ensuring Canada’s Competitiveness”*.

SCN recommends that the Government of Canada provide stable and predictable funding for the Stem Cell Network to maintain operations after March 31st, 2019.

Introduction

It’s been said that if Canada’s sport is hockey, its science is stem cells. Canada’s leadership in stem cell research dates back to the early 1960s, when the field was defined by Drs. Jim Till and Ernest McCulloch at Toronto’s Princess Margaret Hospital. For nearly 60 years, Canadian researchers have been at the forefront of international efforts to turn discoveries into treatments or cures. Today, it is a competitive global field, with OECD nations making strategic investments that will result in long-term health and economic benefits. Canada is well positioned in this global race and productivity gains can be realized. However, this requires a sustained commitment and investment in regenerative medicine, an emerging and innovative sector within our nation’s knowledge economy.

Canada is home to leading-edge stem cell research & clinical trials that seek new treatments for patients with:

- | | |
|-----------------------------|---------------------------|
| <i>Autoimmune disorders</i> | <i>Kidney disease</i> |
| <i>Blood disorders</i> | <i>MS</i> |
| <i>Brain injury</i> | <i>Osteoarthritis</i> |
| <i>Type 1 Diabetes</i> | <i>Sepsis</i> |
| <i>Heart disease</i> | <i>Vision impairments</i> |

The Health & Economic Imperative

Stem cell research is powering regenerative medicine and offering hope for millions of people who live with chronic illness and disease. “The appeal of regenerative medicine lies in its curative approach, which involves treating the causes of a range of conditions by targeting the repair of damaged tissues or organs themselves.”¹

Countries such as Japan, the U.K. and the United States are all vigorously pursuing stem cell R&D in the hopes of leading the field globally. They recognize the potential stem cell research holds for lowering costs associated with chronic diseases – a figure valued at \$190B each year in Canada alone. By developing and commercializing treatments within our borders, Canada can reduce these costs, while spurring productivity.

Current estimates suggest the global market for RM will reach \$66B by 2022. This is a market where Canada is well positioned to capture a significant share, if it moves deliberately and quickly. The foundational pieces are already in place, including a strong and collaborative science culture,

¹ Building on Canada’s Strengths in Regenerative Medicine, March 2017, CCA Report

world-class research institutions, a responsive regulatory environment, a highly skilled workforce; and an open business environment. The challenge lies in maintaining that foundation so that the right conditions continue to exist and encourage business growth built on high-quality science.

Our competitive standing is based on a strong scientific foundation and the vision of leaders capable of moving it along the translational pipeline. The innovations we are commercializing in 2018 are the result of work carried out over the past three decades. However, novel therapies and disruptive technologies that will benefit society will only progress if there is sustained support in place. Stable and predictable funding is needed for research to transition from ‘bench to bedside’ and to spur commercial innovation. This can only be done with the right mechanisms in place. SCN, as the *only* national network and funder for stem cell research, has a proven track record in moving discoveries across the pipeline and into the clinic. With our partners from industry, the charitable and research sectors, SCN is well positioned to deliver stem cell therapies for diseases such as: type 1 diabetes, sepsis, heart failure and retinal degeneration.

**SCN Catalyzing Company Creation:
ExCellThera**

Montreal's ExCellThera is a Canadian startup using a patented cell expansion protocol to provide better outcomes for patients with blood diseases. It was realized with more than \$9M in research funding from SCN between 2003-2018. Recently, this support led to positive initial results from the company's first clinical trial, which has positioned ExCellThera for success in a competitive global market.

The Regenerative Medicine Ecosystem

Since its inception in 2001, SCN has been leading and building Canada’s stem cell research community. It has stimulated the growth of regional organizations, research networks and activities operating with a shared goal of improving the health and economic well-being of Canadians. Without SCN a national approach for building the sector will be lost.

SCN and its 17 years of success are part of the fabric that makes up Canada’s knowledge economy that is the engine of growth in the 21st century. SCN has supported 169 Canadian research groups or 5,000 highly-skilled FTEs; invested over \$100 million in transformative research, leveraged nearly \$100M in partner contributions; and catalyzed 18 clinical trials and 17 biotech start-ups. In addition, SCN has provided training for thousands of early career researchers across Canada. **Today, SCN has \$60 million in written commitments from partners to support high potential research projects over the next five years.** This is a clear indication of the strong potential of the field and an acknowledgment of the central leadership role SCN plays within the regenerative medicine sector.

Canada's Scientific Strength

Canada's knowledge economy includes the best and brightest regenerative medicine researchers from coast to coast. They are developing the novel therapies for the benefit of Canadians and people around the world, and are able to do so because the Government of Canada provided targeted funding for SCN. SCN is proud to have leveraged this to support the work of internationally respected researchers such as:

Dr. Freda Miller, a stem cell biologist at SickKids Research Institute. Her research focuses on leveraging her discovery that a common diabetes drug, metformin, stimulates the production of new brain cells in children who have undergone treatment for brain tumors. SCN is supporting her research to launch similar trials in children and teens with MS and cerebral palsy.

Dr. Lucie Germain, a tissue engineer at the Université Laval. SCN supports her work using stem cells to create replacement or reconstructed organs and tissues for the skin and eye. Her research resulted in two ground-breaking clinical trials in Canada. The first uses laboratory-grown cornea to treat stem cell deficits in the eye. The second uses reconstructed bi-layered skin for severely burnt patients.

Dr. Tim Kieffer at the University of British Columbia and **Dr. James Shapiro** at the University of Alberta have developed complementary methods to replace deficient beta cells in the pancreas that are responsible for type 1 diabetes. Both approaches involve the use of an encapsulation device implanted under the skin to provide a safe barrier for the replacement cells to produce necessary insulin. SCN is supporting clinical trials for both researchers.

Regenerative Medicine Biotech Sector

Canada is home to a burgeoning regenerative medicine commercial sector with active biotech companies operating across the country. Many have been seeded from the leading-edge scientific work conducted by Canada's researchers, and some, such as BlueRock Therapeutics, are selecting Canada as the location to set up global offices. This is due to the solid reputation and scientific infrastructure we have built in this country, which SCN has helped foster through active partnerships and targeted research support. SCN's partnerships include larger companies such as Vancouver-based STEMCELL Technologies, an international provider

Making Canada a Magnet for Biotech

SCN support helped establish Toronto as a leading biotech hub, with more than 75 such companies operating in the region. In 2016, backed by \$225M in venture capital, BlueRock set up key offices in Toronto. Other recent ventures in the city include Johnson & Johnson's first international JLABS incubator, and start-ups PanCella and Sartorius. SCN's pan-Canadian efforts have also planted the seeds for similar sector growth in Vancouver, Montreal and Ottawa.

of tools and technologies that employs more than 1,000 globally, and smaller companies at the cutting edge, such as Aspect Biosystems, an award-winning firm that specializes in 3D bioprinting and tissue engineering.

In a one-day summit organized by SCN in June 2017, a life sciences venture capitalist noted that investors are increasingly looking to Canada for new opportunities, due to our ability to move research from ‘bench to bedside’ and a competitive framework for clinical trials that makes the country an attractive option for investment.

Canada is well positioned to compete in this landscape by incubating many more stem cell and regenerative medicine companies and technologies – but only if there is a supportive ecosystem that can move research across the pipeline to the clinic and marketplace. This ecosystem has to include support for clinical trials and training for HQPs.

Training Canada’s Highly Skilled Workforce

Future competitiveness in an increasingly knowledge-based economy will depend on the skills and expertise of the next generation. SCN and its partners provide specialized training that ensures early career investigators are well placed to compete in Canada’s knowledge-based economy.

A recent study of 10,000 PhDs found that increasingly, PhD graduates from the life sciences (21%), are finding employment in the private sector and major employers are in the biotechnology and pharmaceutical area. This trend is expected to be consistent for regenerative medicine, as companies such as STEMCELL Technologies are planning to expand to over 3,000 employees (an increase of 2,000) in the coming years. Regenerative medicine trainees possess the leading-edge STEM skills critical for innovating in a world where science and technology now dominate. Since 2001, SCN has provided over 7,000 training opportunities to approximately 2,500 individuals from across Canada. SCN’s trainees, like Nika Shakiba and Ben Paylor (see callout box) are making an important difference today and will be critical contributors in the years to come. It is important to note that without SCN in place advanced and targeted training would be lost, as no other organization within the sector has a national mandate for training HQP.

The SCN Trainee Advantage

Ben Paylor left academia for the corporate sector after receiving his PhD in stem cell biology. He is an entrepreneur specializing in science communication and a business consultant. He credits SCN training for providing exposure to a wide variety of problem-solving opportunities, leadership positions and a vast network of researchers, all invaluable to his current success.

“It is because of my experience as an SCN trainee that I have been able to build technical skills relevant for working in a lab, and also establish a network of peers from across Canada and internationally who I can collaborate with and learn from.” - Nika Shakiba, former SCN trainee in Biomedical Engineering

Clinical Trials

Clinical trials are an essential, yet costly step on the road to commercialization. They ensure that new treatments, therapies and technologies are safe and effective before they are widely available. Nonetheless, there are barriers for academic researchers who want to pursue a clinical trial, including lack of funding and expertise in early phase clinical trials that can result in promising developments being sidelined. SCN has established itself as a vital conduit by supporting researchers in the key translational steps and by providing targeted funding for early-stage clinical trials.

SCN's goal is to build a foundation of support to enable 'shovel-ready' clinical trials to move forward, but to also create an environment that will help to accelerate and increase their success rate. This support includes innovative awards and access to recommendations from regulatory, venture capital, reimbursement and patient leaders on SCN committees. Without this kind of robust clinical trials environment, Canada's competitive clinical advantage will be hindered and Canadians will seek care in other countries where access to novel treatments is readily available, but safety standards vary.

Conclusion

A competitive knowledge economy is dependent on a robust science and technology enterprise, one where highly skilled workers are able to generate the knowledge, discoveries, technologies and other innovations. Today, there is clear evidence that the strategic investments made by SCN in stem cell research and regenerative medicine are paying off.

Canada is at a tipping point. Without stable and predictable funding for stem cell research, we will lose out to more ambitious nations. They will be the ones who will capture global market share and develop supporting technologies. Theirs will be the people benefiting first from new and better health care options. Now is the time to 'double down' on the investments already made and commit to the future of stem cell research. Continued support for the Stem Cell Network will benefit the knowledge economy and Canadian competitiveness on the world stage, while improving the health and well-being of its citizens.