



AUDIT AND EVALUATION BRANCH

Evaluation of the Stem Cell Network

REPORT

March 2021



This publication is available online at https://www.ic.gc.ca/eic/site/ae-v-e.nsf/eng/h_03940.html

To obtain a copy of this publication, or to receive it in an alternate format (Braille, large print, etc.), please fill out the Publication Request Form at www.ic.gc.ca/publication-request or contact:

ISED Citizen Services Centre
Innovation, Science and Economic Development Canada
C.D. Howe Building
235 Queen Street
Ottawa, ON K1A 0H5
Canada

Telephone (toll-free in Canada): 1-800-328-6189
Telephone (international): 613-954-5031
TTY (for hearing impaired): 1-866-694-8389
Business hours: 8:30 a.m. to 5:00 p.m. (Eastern Time)
Email: ISED@Canada.ca

Permission to Reproduce

Except as otherwise specifically noted, the information in this publication may be reproduced, in part or in whole and by any means, without charge or further permission from the Department of Industry, provided that due diligence is exercised in ensuring the accuracy of the information reproduced; that the Department of Industry is identified as the source institution; and that the reproduction is not represented as an official version of the information reproduced, or as having been made in affiliation with, or with the endorsement of, the Department of Industry.

For permission to reproduce the information in this publication for commercial purposes, please fill out the Application for Crown Copyright Clearance at www.ic.gc.ca/copyright-request or contact the ISED Citizen Services Centre mentioned above.













© Her Majesty the Queen in Right of Canada, as represented by the Minister of Industry, 2021.

Cat. No. Iu4-405/2021E-PDF

ISBN 978-0-660-38985-1

Aussi offert en français sous le titre *Évaluation du Réseau de cellules souches*

Table of Contents

		<u>Select Page</u>
	Background	3
	About the Evaluation	9
	Findings	14
	EQ 1: Relevance	15
	EQ 2: Networking & Collaboration	18
	EQ 3: Outreach & Engagement	21
	EQ 4: International Standing	25
	EQ 5: Training	27
	EQ 6: Translational Research	31
	EQ 7: Efficiency	34
	Conclusion	38
	Sources & Annexes	39



Background

- *Program Overview*
- *Program Activities*
- *Program Funding*

Regenerative Medicine and Stem Cell Research

Stem cells are special human cells that are able to develop into many different cell types and can renew themselves, allowing the body to heal. Their existence was first demonstrated by two Canadian scientists, James Till and Ernest McCulloch in the 1960s at the Princess Margaret Hospital in Toronto.

Regenerative medicine is the branch of medicine that develops methods to regrow, repair or replace damaged or diseased cells, organs or tissues – including the generation and use of therapeutic stem cells, tissue engineering and the production of artificial organs. A **stem cell therapy** is any treatment that uses stem cells as the primary way of curing or reducing the severity of a disease or disorder. Stem cells have been used to treat leukemia, multiple myeloma and other blood cancers. They also have the potential to treat respiratory diseases, heart disease, diabetes, Crohn’s disease, Parkinson’s, and many others.

The ‘Global Regenerative Medicine Market Analysis & Forecast to 2025’ report estimated that the global regenerative medicine market was worth US\$35 billion in 2019, and will grow to over US\$124 billion by 2025.¹ According to the Alliance for Regenerative Medicine, global financing for the first three quarters of 2020 for the regenerative medicine sector set an annual record of US\$15.9 billion, including US\$8.5 billion in public financing.

Stem Cell Research

There are three stages to Stem Cell research: (1) fundamental/basic research; (2) translational research; and (3) clinical applications.

Translational research is the process of applying knowledge from basic research and clinical trials to techniques and tools that address critical medical needs (clinical applications). The ‘**Translational Gap**’ describes how promising basic research findings fail to make their way into (or out of) clinical trials – due to lack of funding/investments – and therefore never have a chance to develop into therapies for patients.

The **Stem Cell Network** addresses the translational gap by helping to support multidisciplinary research projects move beyond clinical trials and into application.

Stem cell-based therapies and technologies have the aim of improving health and lowering healthcare costs associated with chronic diseases.

Stem Cell Network
2015-16 Annual Report

The Stem Cell Network

The Stem Cell Network (SCN) is a national, not-for-profit corporation **dedicated to enabling the translation of stem cell research into clinical applications, commercial products and public policy.** The network was established in 2001 through the Government of Canada's Tri-Council Networks of Centres of Excellence (NCE) program.

In 2016, funding to the SCN transitioned from the NCE to Innovation, Science and Economic Development Canada (ISED). ISED's contribution to the SCN (a \$36 million commitment over 6 years starting in 2016-17) is geared towards the long-term outcome of **advancing stem cell research to strengthen Canada's economy and the health of Canadians.**

Short-Term Outcomes



Outreach and Engagement: increase the breadth and depth of knowledge related to stem cells, including their ethical, legal and social implications (ELSI) for public policies and practices.



Networking: create national and international partnerships that bring together key individuals and organizations in the stem cell field.

Medium-Term Outcomes



Training: develop a pool of Highly Qualified Personnel (HQP) trained to undertake future stem cell research.



Translational Research: support world-class stem cell research and the translation of that research into clinical applications and commercial products.



International Standing: strengthen Canada's international leadership and reputation in the stem cell field.

Stem Cell Network – Stakeholders and Governance

Stakeholders

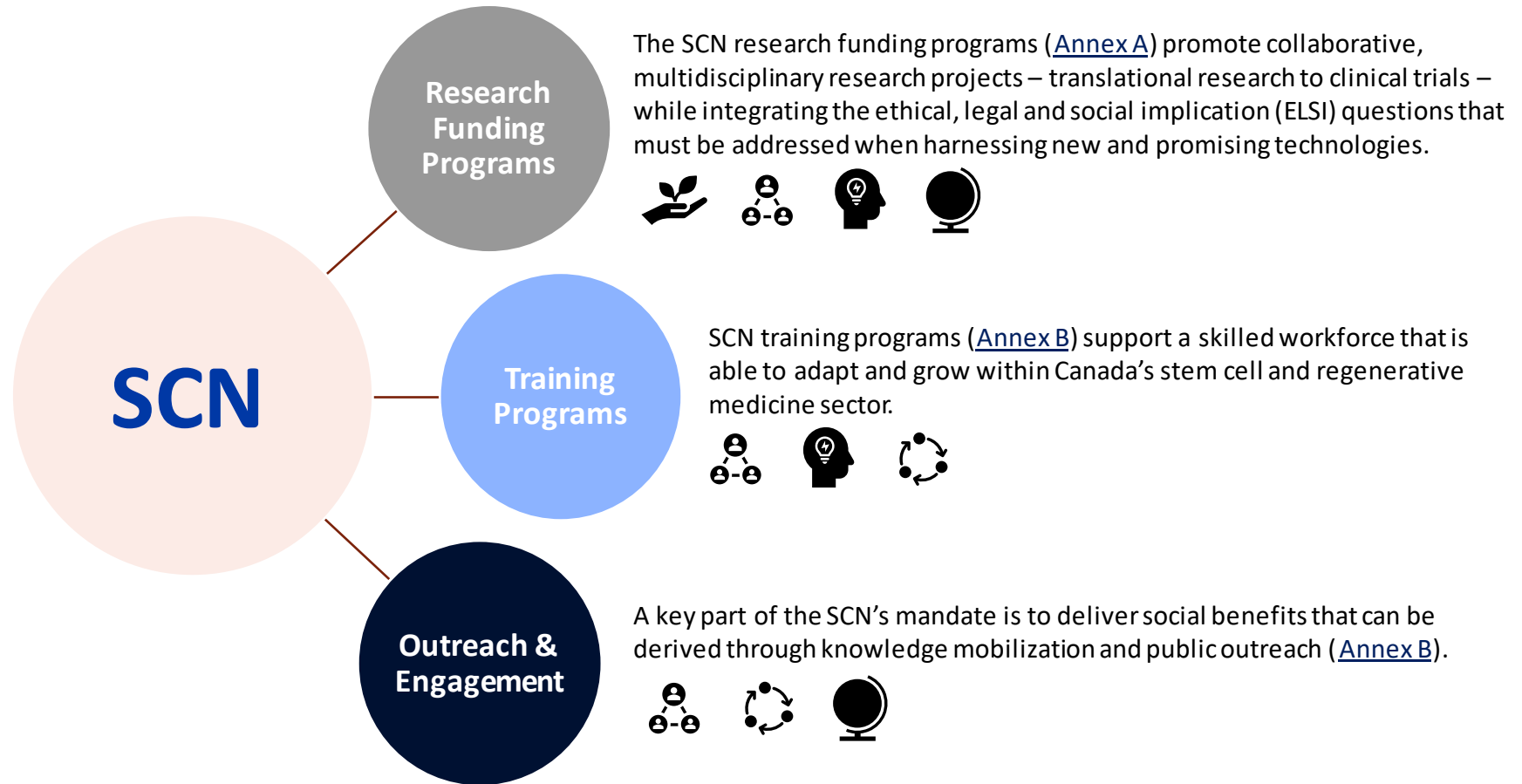
Organizations and individuals participating in the delivery of the SCN's mandate and those directly or indirectly benefitting from its delivery include:

- **Stem cell researchers:** Researchers develop proposals for funding that they submit to the SCN in response to calls for proposals and those who are awarded funding through a peer reviewed process undertake translational stem cell research, with a path to clinical application or commercialization. This includes the stem cell research community at Canadian universities, research hospitals, and not-for-profit research institutions.
- **Post-secondary institutions and affiliated research hospitals:** Institutions host stem cell researchers and assist the SCN in the administration of the program and the transfer of funds to researchers and others directly engaged in eligible activities of the research projects supported by the SCN.
- **Partners:** Organizations such as not-for-profits, health charities, non-federal government organizations and industry provide cash and/or in-kind contributions to SCN-funded projects.
- **Users:** Stakeholders from not-for-profits, industry and the public sector (e.g., Health Canada) who make use of the knowledge generated from stem cell research.

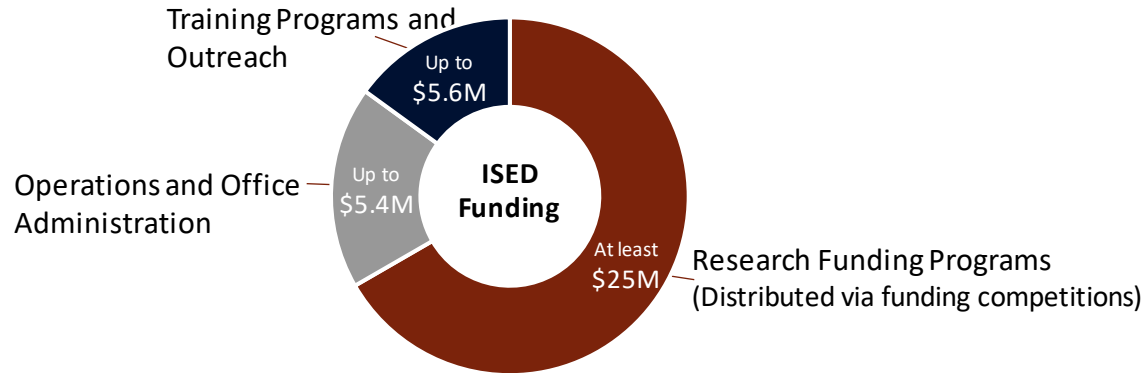
Governance

- The SCN has six full-time staff, including a Scientific Director and Chief Executive Officer, an Executive Director and Chief Operating Officer, and other directors and managers responsible for research and partnerships, communication and administration.
- The SCN is overseen by a Board of Directors, who are responsible for confirming that scientific funding recommendations presented have supporting scientific advice provided by the Research Management Committee and International Peer Review Committee (who review projects for scientific merit).
 - The Board acts in accordance with the requirements laid out by applicable federal and provincial legislation, and its own policies and bylaws. New Board members are nominated by existing Board members and ultimately appointed after approval by SCN members (i.e., research institutions and hospitals) at the annual general meeting.
 - The Board works closely with the SCN management team to support the overall success of the SCN in achieving its vision and mandate.

The SCN supports research funding programs, training programs, and outreach and engagement activities to achieve their outcomes.

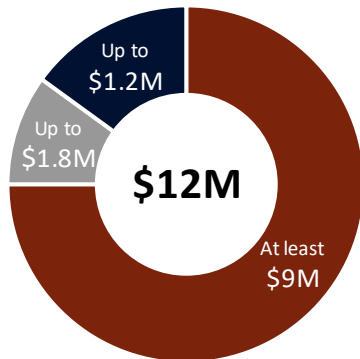


The SCN allocates funding to its activities according to its Contribution Agreements with ISED. Since 2016-17, ISED has committed to providing \$36 million in funding to the SCN over 6 years.



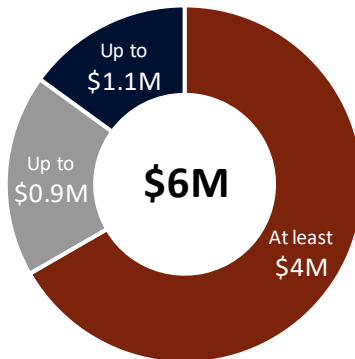
* The SCN received \$83.3 million in NCE contributions between 2001 and 2016.

Contribution Agreement #1
2016-17 to 2017-18
(2 years)



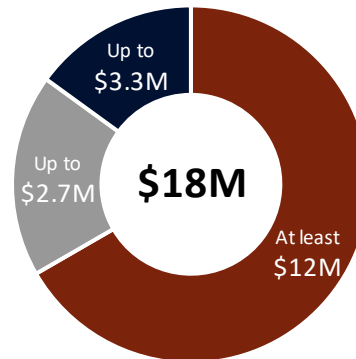
Funding Competition #1
(Sept. 2016 – Dec. 2017)

Contribution Agreement #2
2018-19
(1 year)



Funding Competition #2
(Apr. 2018 – Feb. 2019)

Contribution Agreement #3
2019-20 to 2021-22
(3 years)



Funding Competition #3
(Jan. 2020 – Jan. 2022)



About the Evaluation

- *Evaluation Context*
- *Evaluation Questions*
- *Data Collection*

An evaluation of ISED's contribution to the SCN is a requirement under the *Financial Administration Act*.

The objectives of this evaluation are to examine the relevance, performance and efficiency of ISED contributions to the SCN in accordance with the Treasury Board Secretariat *Policy on Results*. The evaluation covers the period from April 1, 2016, to March 31, 2020.

This evaluation maximizes the use of secondary research (program documentation, literature and pre-existing data), and makes targeted use of primary research (interviews) to focus on the progress toward the short- and medium-term outcomes identified in the SCN logic model ([Annex C](#)). Given that ISED funding to the SCN began on April 1, 2016, the long-term outcomes of the SCN (i.e., advancements in stem cell research strengthen Canada's economy and health of Canadians), were not a focus of this evaluation as it typically requires an elapsed time period of at least five years to assess.

Previous Evaluations

Prior to the 2016 transition in funding from the NCE to ISED, NCE funding to the SCN was provided via competitive grants on a project-by-project basis.

While there has been no evaluation conducted for the SCN, there were two mid-term reviews completed while the SCN was under the NCE, which assessed the SCN as having strong governance and generating clear benefits based on the calibre and output of its research.

Relevance



1. To what extent is there a continued need for stem cell research?
 - a. To what extent does the SCN address a unique need?

Performance



2. To what extent has the SCN contributed to increasing networking and collaboration among researchers from Canada and abroad?



3. To what extent has the SCN contributed to increased breadth and depth of knowledge related to stem cells, including the ethical, legal and social implications of stem cells?



4. To what extent has the SCN contributed to enhancing Canada's international profile and visibility in stem cell research and clinical trials?



5. To what extent has the SCN contributed to Canada having a solid base of researchers that are trained to undertake future stem cell research?



6. To what extent has the SCN contributed to stem cell research being translated into clinical application and commercial products?

Efficiency



7. To what extent has the SCN been an efficient model for supporting stem cell research in Canada?

The evaluation relied on multiple lines of evidence to address program relevance, performance and efficiency, including both qualitative and quantitative research methods.

Document Review

The document review included ISED program foundational documents; government priority-setting documents; SCN annual reports, corporate and strategic plans; and SCN-commissioned or completed studies.

Literature Review

The literature review identified and extracted relevant information and data from websites and other sources such as grey literature, funder announcements, etc.

Interviews

A total of 42 telephone interviews were conducted with stakeholders representing the following six groups:

- SCN researchers (14);
- SCN trainees (6);
- SCN officials and Board members (6);
- Canadian companies/health charities partnered with the SCN (5);
- Post secondary institutions/affiliated research hospitals (8); and
- ISED program management (3).

Data Review

The data review included the SCN's administrative and operational data to assess the efficiency of program delivery.

Evaluation Limitations and Mitigation Strategies

The evaluation encountered some limitations and applied the mitigation techniques outlined below.

Attribution

The presence of other funding partners (e.g., other levels of government, private sector, etc.) made it difficult to isolate and measure the impact of the federal government's contribution.

- To mitigate this, interview questions were designed and articulated in a way that respondents could answer, to the extent possible, the incremental impact of ISED's funding to the SCN. The interview findings were then triangulated with findings from the document review and data review.

Respondent Bias

Another challenge and limitation associated with this evaluation was the potential for respondent bias. For example, some of the interviewees are involved in program design and delivery or are funding recipients. As such, the findings may be biased towards more favourable program outcomes.

- To mitigate this, the purpose of the interview and strict confidentiality was clearly communicated to participants and responses were cross-validated across stakeholder groups.

Assessment of Impact

Research projects often require a longer timeframe to show a tangible impact on the sectors and/or communities they study, thereby presenting a limitation to fully assess the possible socio-economic impacts of SCN-funded research.

- As a mitigation technique, whenever possible, anecdotal evidence of impacts was collected through the interviews and available SCN impact reports.



Findings

- *Relevance*
- *Performance*
- *Efficiency*

EQ1

KEY FINDING: There is a continued need for collaborative stem cell research due to its potential to treat chronic and degenerative diseases as well as the resulting economic benefits.

Need to Support Stem Cell Research in Canada

Stem cell research is an area of strategic strength for Canada. Aside from the discovery of stem cells by Canadian researchers, recent years have seen scientific advances and emerging experimental therapies in stem cell research and regenerative medicine, including the isolation and use of pluripotent stem cells (which can differentiate into any cell type) from human embryos in 1998, and the discovery of induced pluripotent stem cells (created by reprogramming adult stem cells) in 2006. These innovations have led to new opportunities to treat degenerative conditions, revolutionize drug screening, and advance human toxicology research.²

With a global aging population, many countries, including Canada, recognize the potential of stem cell research and regenerative medicine to lead to innovative therapies and treatments for chronic and degenerative diseases such as type 1 diabetes, blood cancer, and Parkinson's, ultimately improving the health of Canadians.

- For example, the work of Canada's Dr. Harold Atkins using stem cell transplants has been proven to stop (and in some cases reverse) the progression of early aggressive multiple sclerosis in some patients.
- Interviewees noted that the benefits of years of stem cell research are only now beginning to be realized.

All 42 stakeholders interviewed indicated that there is a continued need to support stem cell research for the health-related reasons outlined above and also for the following potential economic benefits:

- Reducing Canadian health care costs for treating chronic and degenerative diseases.
- Creating valuable intellectual property (IP) from disruptive technologies.
- Increasing employment of highly qualified personnel (HQP) via new Canadian ventures and companies.

Literature suggests that Canada's position in the stem cell and regenerative medicine field is among the top 5 in the world and is strengthened by a collaborative culture across institutions, disciplines, and provincial borders, which has been fostered by the SCN and its leadership and coordination with other networks in Canada:³

- Centre for the Commercialization of Regenerative Medicine (CCRM): focused on the development and commercialization of therapies and technologies.
- CellCAN: focused on manufacturing practices.
- Ontario Institute of Regenerative Medicine and ThéCell in Quebec: provincial organizations.

To what extent is there a continued need for stem cell research?

To what extent does the SCN address a unique need?



EQ1

To what extent is there a continued need for stem cell research?

To what extent does the SCN address a unique need?



KEY FINDING: The SCN is the only national organization in Canada with a sole focus on supporting stem cell research from beginning to end and the training and development of future stem cell researchers. However, the time-limited nature of ISED-SCN funding agreements can impact the SCN's ability to secure long-term funding from other sources and develop longer-term programs with the potential to generate greater benefits.

Stem Cell Network Addresses a Unique Need

The SCN's role and objectives help advance government priorities. It aligns with the Innovation and Skills Plan's goal to increase collaborations between research institutions and industry to help maintain Canada's leadership role in scientific research. Further, priorities outlined in the Minister of Innovation, Science and Industry's mandate letter highlight a continued need to support investing in scientific research, including new discoveries and the commercialization of ideas.

According to the literature and interviews, the SCN is the only national organization focused on stem cells that fosters a research culture and community that embraces a multidisciplinary and collaborative approach (i.e., bringing expertise from various disciplines together), and targets its funding towards high-quality, collaborative research, both of which help leverage partner funding.

- Interviewees noted that stem cell research is not a key priority for granting councils such as the Canadian Institutes of Health Research (CIHR), which focuses its funding on fundamental research and more broadly-focused clinical-based research. The SCN also follows projects from start to end, providing guidance through its Research Management Committee, which provides additional scientific direction to help reach positive outcomes and ensure effective use of funding.

Interviewed trainees stated that the SCN is the only national network that financially supports stem cell trainees to travel across Canada for training and networking opportunities, both essential for career development. The training and support provided by the SCN is critical for understanding the steps required for moving fundamental research into future therapies and potential commercial products. It is also essential for ensuring that research capacity in Canada is maintained and HQP is retained.

The SCN provides a liaison between researchers and the federal government, including interacting with federal government departments (e.g., Health Canada, ISED).

- Researchers benefit from the SCN's connections and expertise and their ability to influence decision making.

EQ1

To what extent is there a continued need for stem cell research?

To what extent does the SCN address a unique need?



Further, the SCN helps provide visibility to stem cell researchers internationally.

- The SCN is in direct contact with stem cell related organizations in other countries (e.g., US, UK and Australia) and this helps provide exposure to Canadian researchers and their research.

Funding Stem Cell Research

The literature acknowledges Canada as one of the global leaders in stem cell and regenerative medicine research. Other global leaders include the U.S. (California in particular), U.K., Japan, South Korea and Singapore – each of whom provide funding for stem cell in their countries.⁴

- For example, as of late 2019, the California Institute for Regenerative Medicine (CIRM) had awarded about \$2.7 billion in grant funding since its inception in 2004. In 2020, an additional \$5.5 billion was allocated to the CIRM. By comparison, approximately C\$705 million was invested publicly in Canadian stem cell research from 2001 to 2017⁵.

The literature and document review revealed that financial gaps have been inherent challenges for Canada’s commercialization efforts, particularly since exploring the potential of new therapies (notably proof of principle research and phase I/II clinical trials) requires large amounts of funding over long and uncertain timeframes. As a result, many investors are reluctant to engage in this sector due to uncertainties related to clinical efficacy, intellectual property, and untested regulatory pathways.

- The document review found that a challenge with stem cell research is keeping the research pipeline stabilized and full, so that new stem cell based therapies, treatments and technologies are realized well into the future.

The majority of interviewees suggested that funding stability is crucial for translational research, as it can support scientific advances to the point where the degree of risk is acceptable to private organizations.

- The literature noted that the SCN has been successful at leveraging multiple sources of funding from federal, provincial, non-governmental and industry sources.

Interviewees noted that the SCN addresses a unique need in funding translational research, but that without stable funding for stem cell research (e.g., via the SCN), momentum could be lost, promising therapies could halt, and the ability to secure long-term funding from other sources could be lost. Additionally, there is the potential for Canada to lose its pipeline of HQP to competing countries, as well as the IP it has developed, ultimately risking Canada’s international standing and leadership in the stem cell field.

EQ2

KEY FINDING: The SCN has helped increase networking and collaboration among researchers domestically and internationally, particularly through the annual Till and McCulloch Meeting and training and workshop events. In addition, the multidisciplinary requirements of SCN-funded projects enhances networking and collaboration.

To what extent has the SCN contributed to increasing networking and collaboration among researchers from Canada and abroad?



Networking in Canada and Abroad

There was unanimity among SCN-funded researchers, partners and trainees that the SCN has contributed to increasing networking in Canada and abroad due to it being the only national facilitator for stem cell research collaboration.

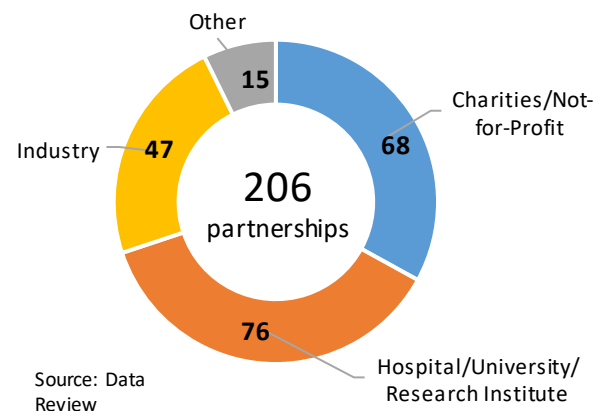
All stakeholders interviewed noted that the main vehicle for increasing networking in the SCN is the annual Till and McCulloch Meeting, which typically attracts hundreds of participants from Canada and abroad. The meeting helps facilitate networking among people with different backgrounds and leads to the forming of research collaborations.

The SCN partners with industry, government, and the not-for-profit (NFP) sector to provide support for stem cell and regenerative medicine research collaborations, outreach, and training opportunities at various events and workshops, in Canada and abroad.

Partnerships in Canada

The document review indicated that the SCN contributes to increasing networking through its funding programs, which require collaborations based on jurisdiction, diversity and field of expertise.

- SCN researchers noted that the multidisciplinary requirements encourage researchers to expand beyond their usual networks and leads to the creation of well-balanced teams that move high-quality research forward faster.



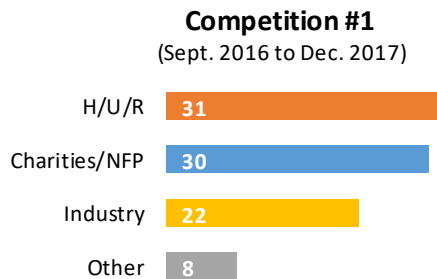
During the evaluation period, 68 SCN-funded projects led to 206 partnerships (providing cash or in-kind support) between hospitals/universities/research institutes (H/U/R), charities/NFP, industry and other organizations.

EQ2

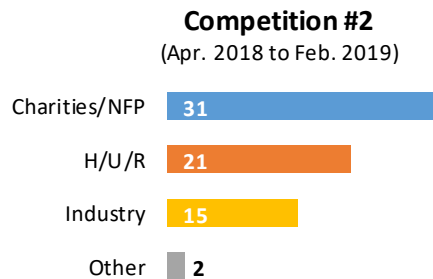
To what extent has the SCN contributed to increasing networking and collaboration among researchers from Canada and abroad?



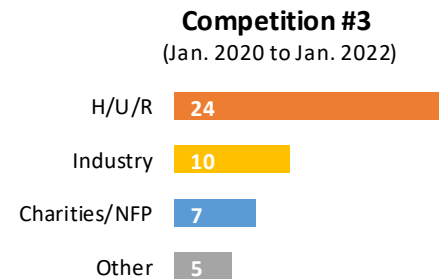
SCN-Funded Research Partnerships by Funding Competition



Source: SCN Administrative Data



Source: SCN Administrative Data



Source: SCN Administrative Data

Partnerships with Charities/NFP and/or H/U/R were the most pervasive.

Collaborations in Canada

The 68 SCN-funded projects included:

- 68 funded lead investigators and 74 funded co-investigators, totaling 142 SCN-funded collaborations.
 - Male and female collaborators represented 68% and 32% of the 142 funded collaborators, respectively.
 - 3.5% of funded collaborators self-identified as a visible minority.
- An additional 300 collaborating investigators (not directly funded by the SCN) and 737 highly qualified personnel (HQP).
 - Male and female investigators represented 76% and 24% of the 300 collaborations, respectively.
 - 2% of the collaborating investigators self-identified as a visible minority.
 - The majority of the 737 HQP (56%) self-identified as female, while 7.7% self-identified as a visible minority.

EQ2

To what extent has the SCN contributed to increasing networking and collaboration among researchers from Canada and abroad?



Collaborations Abroad

On an international level, as of March 31, 2020, 35% of all SCN-funded projects (24 of 68 projects) included at least 1 international collaboration, with 33 international collaborators in total.

- 20 of the 33 international collaborators were with the U.S.

A bibliometric study⁶ examined 304 SCN-supported publications from 2013 to 2020. The 304 publications consisted of 1,766 individual authors from Canada and abroad. In total, about one-third of all authors were international, with the U.S. making up about half (50.4%) of all international collaborators.

Country	Distribution of International Collaborators
United States	50.4%
United Kingdom	7.0%
Australia	5.3%
Germany	4.6%
China	3.7%
28 countries	<3.0%

Source: SCN Administrative Data

EQ3

KEY FINDING: The SCN continues to expand the breadth and depth of knowledge related to stem cells and the ethical, legal and social implications, by funding innovative research projects, supporting research publications in high impact journals, and sponsoring SCN researchers to present their findings at national and international workshops and conferences.

The SCN contributes to increased breadth and depth of knowledge by funding high quality research and supporting outreach and engagement, including online activities.

Funding High Quality Research

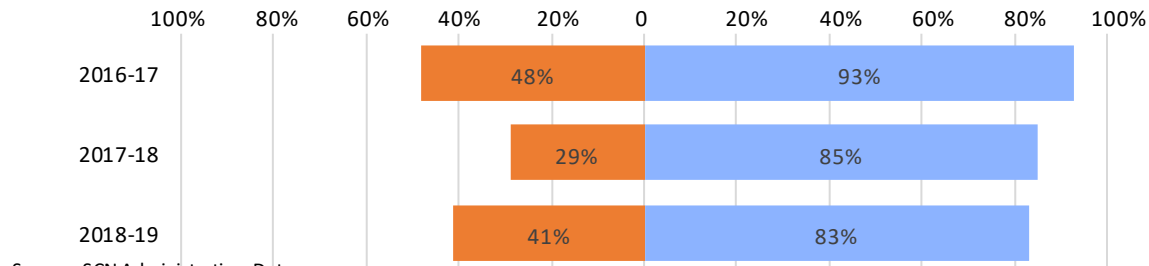
Most SCN researchers interviewed indicated that the SCN contributes to the production of high quality and transformative stem cell research via the prerequisite that projects are multidisciplinary – more transformative research is possible by bringing different sets of expertise together, which leads to higher quality research.

Interviewees noted that the SCN is strategic in the research they support, by implementing a high-level international peer review process for all project applications, focusing on how the research project can be performed, the quality of the research, and how it can translate to next steps. This highly regarded approach has enabled the SCN to leverage partner and industry funding, which is necessary to advance pre-clinical research to clinical trials.

According to the SCN administrative data, there were 202 SCN-supported publications between September 2016 and February 2019.

- The 202 publications had 82 (40%) female lead authors, and 174 (86%) were published in scientific journals.

% of SCN publications (202) with a **female lead author**
 % of SCN publications (202) in **scientific journals**



Source: SCN Administrative Data

To what extent has the SCN contributed to increased breadth and depth of knowledge related to stem cells, including the ethical, legal and social implications (ELSI) of stem cells?



EQ3

To what extent has the SCN contributed to increased breadth and depth of knowledge related to stem cells, including the ethical, legal and social implications of stem cells?



Funding High Quality Research (cont.)

Results from the bibliometric study (which covered the period from the end of 2013 to the start of 2020) showed that there were 304 SCN-supported publications—generating 6,442 citations, with more than half (55%) of all publications receiving at least 10 citations.

- 98% of publications appear in journals ranked in either the top or second to top quarter of their respective field.

SCN funding is also targeted at projects focused on the ethical, legal and social implications (ELSI) of innovative stem cell research.

- These projects impact science policy in Canada and examine concerns such as stem cell tourism, informed consent, stem cell banking (i.e., Cord Blood Bank) and embryonic stem cells.
- Interviewees noted that SCN-funded ELSI researchers have become world renowned in the field and, in turn, Canada has benefitted from this expertise by establishing regulations and laws around stem cell research and regenerative medicine (e.g., informing the Assisted Human Reproduction Act).
- During the evaluation period, the SCN funded 6 different ELSI projects, with 5 projects (83%) having a female principal investigator or co-investigator.

Travelling to another country to undergo an experimental treatment can be expensive, ineffective and possibly dangerous.

Knowledge Dissemination and Mobilization

SCN-funded researchers pointed to a number of ways in which the SCN helps to increase the breadth and depth of knowledge related to stem cell research – notably via knowledge dissemination and mobilization:

- 1) The annual **Till and McCulloch meeting** was mentioned by interviewees and noted in the document review as enabling researchers and trainees to connect, network and share research and ideas. Without the meeting, the stem cell research ecosystem would be at risk of becoming fragmented, which can lead more to competition amongst researchers as opposed to collaboration.

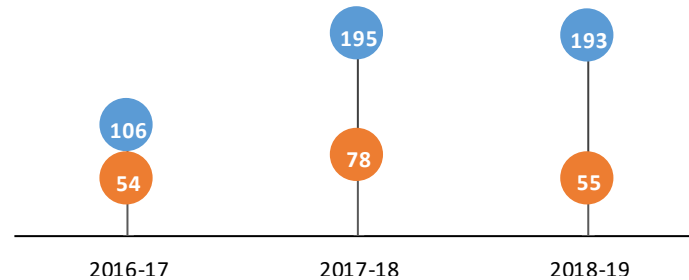
EQ3

To what extent has the SCN contributed to increased breadth and depth of knowledge related to stem cells, including the ethical, legal and social implications of stem cells?



- 2) The SCN supports **presentations at conferences and workshops** for international partners to understand the breadth and depth of stem cell research in Canada.
- From 2016 to 2019, there were 494 SCN-supported investigator presentations at workshops and conferences around the world, with 60% of presentations hosted in Canada, 10% in the U.S., and 30% abroad (in the U.K., France, Japan, Germany, Spain, and elsewhere).

Total number of **presentations** at workshops and conferences (**494**)
 Total number of **female** lead authors of the presentations (**38%**)



Source: SCN Administrative Data

- 3) **StemCellTalks (SCT)** is a national stem cell biology outreach initiative, which allows high school students to spend a day with scientists who are experts in stem cell and regenerative medicine to learn about stem cells and the potential they have for treating diseases such as macular degeneration, diabetes and multiple sclerosis. From 2016 to 2020, SCT hosted 32 events, reaching 3,847 high school students across Canada.

SCN researchers also noted that the workshops allow trainees and researchers to learn different specialized techniques (e.g., flow cytometry, RNA sequencing, bioinformatics, genome editing, etc.) that would otherwise be challenging to learn on their own.

- These workshops also provide information on 'softer' skills such as how to present a talk, how to collaborate and how to navigate the regulatory environment.
- The knowledge and skills obtained in the workshops are then brought back to the researchers'/trainees' respective labs to help improve their research.

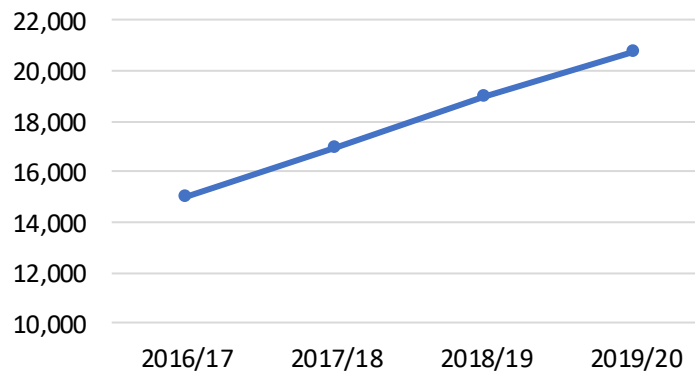
EQ3

Online Activity

The SCN’s website and social media platforms allow the SCN to share stem cell research and ELSI findings, provide accurate information about the potential of stem cells, and inform about events/activities where the public can learn more about stem cells and related research. For example, the SCN has been instrumental in highlighting ‘stem cell tourism’ – a practice among patients to pay for unproven stem cell therapies at private clinics. According to the document review:

- From 2016 to 2020, the SCN’s number of Twitter followers grew from 15,000 to 20,800).
- Over the same timeframe, CellLines (the SCN’s online newsletter) grew from 800 to 1,500 subscribers, an 88% increase.
- From 2018 to 2020, the Stem Cells 101 website views increased by 15%, from 2,566 to 2,938. Over the same timeframe, the Stem Cells Education website views decreased by 4%, from 1,455 to 1,401.

**SCN Twitter Followers
up 39% over 4 Years**



In 2018, the EDI social media campaign entitled “Five to be inspired by” profiled five women in the stem cell and regenerative medicine sector across Canada. The campaign received 37,134 impressions (views) on Twitter – the most in the SCN’s social media campaign history.

To what extent has the SCN contributed to increased breadth and depth of knowledge related to stem cells, including the ethical, legal and social implications of stem cells?



EQ4

KEY FINDING: The SCN has helped enhance Canada's international standing and leadership with respect to fundamental research and translational research. To further enhance Canada's standing, particularly with respect to clinical trials, more targeted resources may be needed.

Canada's International Standing in Stem Cell Research and Clinical Trials

Most interviewees agreed that Canada's international standing in stem cell research can be attributed to the SCN's role in:

- Bringing the global stem cell research community together;
- Funding high-quality research;
- Promoting Canadian stem cell research through conferences in Canada and abroad;
- Training researchers and informing the public (e.g., creating awareness of stem cell tourism);
- Helping develop technology patents;
- Improving the regulatory environment around stem cell research in Canada (e.g., informing the *Assisted Human Reproduction Act*); and
- Supporting Canada's position in the International Standards Organization to ensure the Canadian point of view is included in the development of international standards.

High quality stem cell research funded by the SCN has raised Canada's international profile. For example, SCN-funded research into the use of stem cells for septic shock (infection throughout the body) led to the first ever sepsis clinical trial. As a result, an international meeting with global experts was supported by the SCN to share and harmonize protocols related to stem cell therapies for sepsis.

- According to World Health Organization data, Canadian clinical trials represented 5.2% of global clinical trials during the 1999-2019 period, ranking 8th in the world (the U.S. ranked #1 at 25.6%, with China 2nd at 8.8%).

Most SCN researchers interviewed agreed that Canada is among the top five countries when it comes to stem cell research, but that the standing is not quite as high for clinical trials.

- This viewpoint was supported in a survey⁷ of 60 international stem cell experts from 12 countries, which noted that only the U.S. is seen as a more significant contributor to the stem cell field than Canada, which ranks approximately on par with Japan, and above the U.K. and all other nations. Canada is viewed as being most well-known for discovery/basic science and pre-translation and translational research, considerably more so than commercialization, policy, and clinical trials.

To what extent has the SCN contributed to enhancing Canada's international profile and visibility in stem cell research and clinical trials?

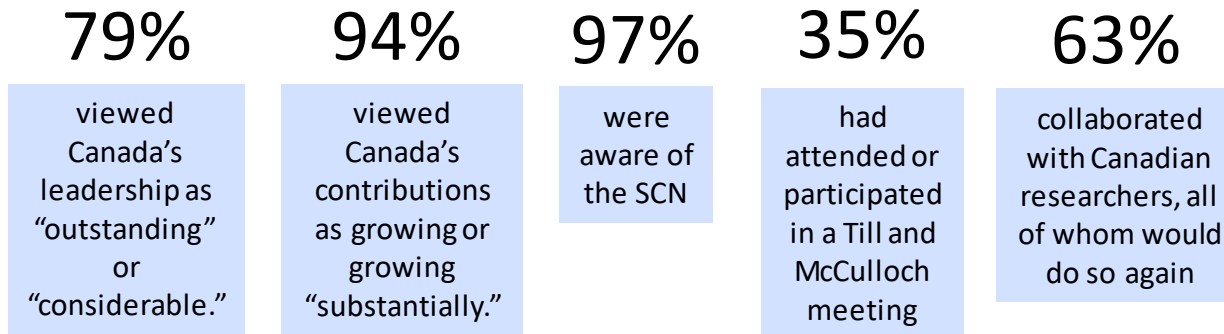


EQ4

To what extent has the SCN contributed to enhancing Canada's international profile and visibility in stem cell research and clinical trials?



The survey of 60 international stem cell experts showed that research supported by the SCN is consistently of strong and exceptional quality, and has considerable impact on the stem cell field. Of the 60 stem cell experts:



Canada's International Participation

The document review noted that the SCN offers support for Canadian stem cell researchers to participate in international meetings such as the International Society for Cellular Therapies (ISCT) and the International Society for Stem Cell Research (ISSCR) to discuss scientific advancements, funding practices and policy issues from an international perspective.

- SCN's participation in these meetings ensures Canadian investigators are able to present their work through talks and abstracts, helping highlight Canada's strength in stem cells and regenerative medicine research.

Suggestions for Further Enhancing Canada's International Standing

Although evidence suggests Canada is highly regarded in stem cell research, interviewees provided suggestions for the SCN to further enhance Canada's international standing, including:

- Additional and longer-term funding to the SCN to foster more international collaborations and seek international partners in clinical trials;
- Hosting international events (e.g., International Society for Stem Cell Research meeting) in Canada to increase Canada's visibility in stem cell research; and
- Further increasing the SCN's focus on translational research, commercialization, and manufacturing, which will help build anchor companies and large, enduring companies.

EQ5

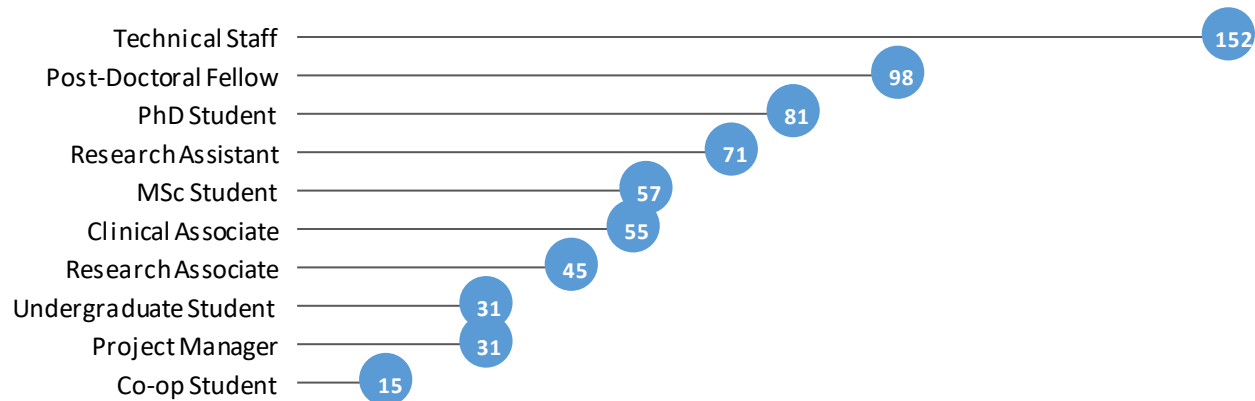
KEY FINDING: The Stem Cell Network enables the career development and research opportunities of trainees, which contributes to a pipeline of qualified researchers that remain in Canada to pursue higher education and employment positions within all areas of Canada’s stem cell and regenerative medicine sector.

Stem Cell Network Enables Career Development and Research Opportunities

The nature of the SCN’s research programs facilitates research opportunities for the development of highly qualified personnel (HQP). For instance, to receive SCN funding, research projects requirements include supporting “dynamic, multi-disciplinary teams that are diverse and reflective of Canadian society and of *all* research career stages”.

- In addition, a trainee education plan must be submitted with research funding applications.
- Trainees noted that the SCN’s support ensures that it is not just students/trainees from more research-intensive labs attending training opportunities - all trainees across Canada have equal opportunity.

The 55 SCN-funded projects from competitions #1 and #2 generated 636 HQP collaboration opportunities during the 2016-17 to 2018-19 period. The largest number of collaboration opportunities was for technical staff.



Source: SCN Administrative Data (excludes collaboration data for competition #3)

During the evaluation period, the SCN supported 1,340 trainees (including a 57% increase from 2016-17 to 2019-20) who have attended or participated in SCN-supported training workshops and courses.

To what extent has the SCN contributed to Canada having a solid base of researchers that are trained to undertake future stem cell research?



EQ5

To what extent has the SCN contributed to Canada having a solid base of researchers that are trained to undertake future stem cell research?



Interviewed partners stated that the SCN and its partners (e.g. CCRM) have played a key role in creating a peer-to-peer support network across Canada. Most notably, support is provided to trainees at the annual Till and McCulloch meeting, where trainees have the opportunity to present their research findings, as well as organize and participate in learning workshops.

- Trainees comprise roughly half of the 400 annual meeting participants.

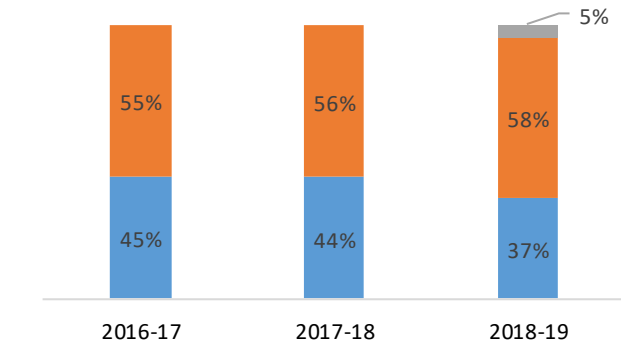
The SCN and its partner organizations offer workshops that provide technical stem cell training (e.g., genome sequencing, flow cytometry, etc.) and more general, ‘soft’ skill training that trainees would not otherwise have access to.

- During the evaluation period (2016-17 to 2019-20), the SCN held 39 workshops.

According to an impact analysis of SCN training programs⁸, the soft skills cited as being most beneficial included general and scientific communication and translational business strategy, which are important skills used in grant writing and commercialization.

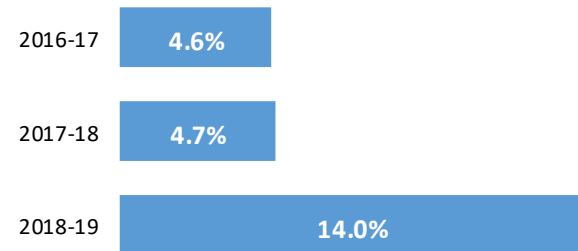
SCN-sponsored events for female researchers (e.g., ‘Women in Regenerative Medicine’ breakfast at the 2018 T&M meeting) were noted as providing an important opportunity to meet with female mentors.

Of the 636 HQP collaborations:
56% self-identified as **Female**
43% self-identified as **Male**
1% had **no response**



Source: SCN Administrative Data

Of the 636 HQP collaborations:
7.7% self-identified as a **Visible Minority***



Source: SCN Administrative Data

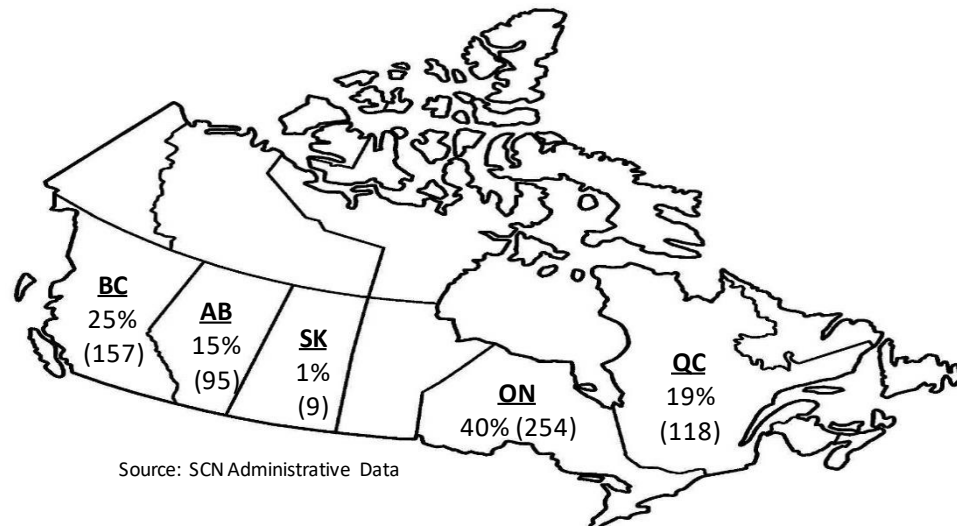
*Formal measures to deal with EDI data collection were implemented in 2018-19.

EQ5

To what extent has the SCN contributed to Canada having a solid base of researchers that are trained to undertake future stem cell research?



636 HQP Provincial Breakdown (includes 3 from the U.S.)



Training Outside of the Stem Cell Network

Interviewees noted that some organizations (i.e., OIRM, ThéCell, BCRegMed, and Medicine by Design in Toronto) offer stem cell research training, but it is usually done in coordination with the SCN and is only regionally delivered.

- Interviewees mentioned that CellCAN offers training, but only for cell manufacturing, which is not covered under the SCNs mandate.

Mitacs, CIHR, NSERC, the Heart and Stroke Foundation, Physician Services Incorporated, and adMare BioInnovations were also mentioned as offering training on topics that touch upon stem cell research, but no other organization aside from the SCN is focused on developing stem cell researchers at the national level to undertake future stem cell research.

Trainees interviewed noted that SCN training is unique and valuable, and is the only organization that provides training opportunities on a national scale.

- Part of the value added by SCN training is that its workshops frequently include new techniques and ideas that may not be covered in school curriculums. They can encompass cutting edge techniques, teach early career researchers how to present ideas, enable networking opportunities, and provide complementary skills training.

EQ5

To what extent has the SCN contributed to Canada having a solid base of researchers that are trained to undertake future stem cell research?



Career Development

Trainees who become members of the SCN training support committee, 'Training Communications Committee (TCC)' liaise between the training community and the SCN's Training Education Committee to tailor workshops/skills development towards the challenges faced by trainees, as well their career development goals. In addition, TCC members learn the logistics of event organizing and fundraising.

Through the SCN workshops, trainees are exposed to all aspects of stem cell research and the stem cell ecosystem, while being provided guidance on how to take the next career steps, helping them understand where job opportunities are, and enabling interactions with companies.

SCN trainees surveyed in the 'impact analysis of SCN training programs' study, which tracked 1,500 HQP supported by SCN training between 2001 and 2019, stated that SCN training is unique and valuable, offering opportunities that would otherwise be unavailable.

- Additionally, trainees interviewed for this evaluation noted that without the SCN's support for conferences and workshops (particularly the Till & McCulloch Meetings), many trainees would look to pursue post-docs and principal investigator positions in other science areas with more funding, and possibly other countries outside of Canada, potentially diminishing the future talent pool of stem cell researchers in Canada.

According to all interviewees, the SCN has had a significant impact on the career development of stem cell researchers in Canada.

- The 'impact analysis of SCN training programs' study found that about 80% of SCN trainees were Canadian citizens or permanent residents at the time of their SCN training, and approximately 82% have since opted to remain in Canada for work following their SCN training.⁹ This suggests that the skills, knowledge and expertise acquired are of high value to industry and lead to meaningful employment in Canada, thereby contributing to strengthening Canada's knowledge economy.

EQ6

To what extent has the SCN contributed to stem cell research being translated into clinical application and commercial products?



KEY FINDING: Through support and targeted funding for translational research, SCN-funded research has led to clinical trials, the development of patent applications, and the establishment and enhancement of start-up companies.

Support for Clinical Trials/Commercialization Activities

SCN researchers provided multiple examples on how the SCN helps researchers translate their research into clinical applications and commercial products, including:

- Organizing and sponsoring workshops to educate on the process of translating research into therapies or treatments.
- Providing financial support for scientific founders of companies to attend investor events.
- Facilitating meetings with industry to go over business plans and provide input on what the potential demand is for a specific product.
- Connecting researchers with patent lawyers, manufacturers and clinical trial designers to help take an idea or concept to the next stage.

Funding Research Programs

SCN officials noted that the SCN's most recent suite of three translational research programs focus on moving research towards clinical trials and the application space:

- **Accelerating Clinical Translation Program:** Project awards of up to \$400,000 to support multi-disciplinary research projects that are moving toward the clinic, as well as translational research activities associated with an upcoming or ongoing clinical trial.
- **Fueling Biotechnology Partnerships Program:** Project awards of up to \$350,000 to support academic partnerships with emerging Canadian regenerative medicine biotech companies who are working to drive an innovative stem cell-based technology or therapy into the clinic or market.
- **Translation and Society Team Awards:** Project awards of up to \$175,000 to support national teams of high-calibre, multi-disciplinary investigators to undertake ELSI research projects that focus on key opportunities and impediments to progress in translation of innovative stem cell research.

SCN researchers noted that the SCN facilitates clinical translation via funding with specific timelines, whereby the SCN proactively helps if things fall behind.

EQ6

To what extent has the SCN contributed to stem cell research being translated into clinical application and commercial products?



Clinical Applications and Commercialization

Clinical Trials

Clinical trials test the safety and efficacy of a new therapy, and are important for detecting, diagnosing, and reducing the chance of developing the disease in question.

Although various SCN supported clinical trials began prior to the evaluation period, 14 clinical trials ([Annex D](#)) received direct investments from the SCN between 2016 to 2020, all of which were successful and proceeded to the next phase of the clinical trial (either phase 1 or 2, with some expected to reach phase 3 within the next two years). Potential clinical applications include, but are not limited to:

Improving	Reducing
Burn wound pain & post-burn scars	Inflammatory response (lungs) in COVID-19 patients
Liver organ transplantation	Fatal septic shock cases
Heart disease repair	Insulin injections for Type-1 Diabetes patients
Corneal health and vision	

Intellectual Property (IP)

Interviewed SCN researchers noted that unlike some other organizations in Canada, the SCN does not take any percentage of the profits generated by IP creation emanating from SCN-funded projects. In addition, The SCN supports researchers by helping fund the costs associated with IP filings as part of eligible research expenses

Between 2016-17 and 2019-20, SCN-funded projects led to:

- 260 patent applications filed (31 in Canada);
- 31 patents issued (2 in Canada); and
- 31 licences signed.

EQ6

To what extent has the SCN contributed to stem cell research being translated into clinical application and commercial products?



Company Development

The SCN’s approach to collaboration and knowledge-sharing have resulted in numerous companies, ranging in size from two employees and less than \$1 million in annual revenue to 1,500 employees and \$270 million in annual revenue. Between 2016 and 2020, 18 companies were either created or enhanced with SCN funding. Bolded companies are highlighted in [Annex E](#).

Created	Enhanced
<p>ExCellThera (Quebec) Inspire Biotherapeutics (Ontario)</p> <p>Morphocell Technologies (Quebec) panCELLa (Ontario) Reveille Inc. (Ontario)</p> <p>Satellos (Ontario)</p> <p>StemAxon (Quebec) TissueX Technologies (Ontario) VisuCyte Therapeutics (British Colombia)</p>	<p>Aspect Biosystems (British Colombia) BlueRock Therapeutics (USA) <i>*Acquired by Bayer in 2019</i> Encellin (USA) Northern Therapeutics (Quebec) Notch Therapeutics (Ontario) Repare Therapeutics (Quebec) Saskin Life Sciences (India) STEMCELL Technologies (British Colombia) ViaCyte (USA)</p>

Company Highlight: ExCellThera

SCN’s funding and approach to collaboration and knowledge sharing between Canadian labs brought together a bioengineer, a hematopoietic stem cell researcher and a chemist, which led to the creation of the Montreal-based company ExCellThera.

ExCellThera aims to increase the quality and quantity of healthy blood stem cells available to treat people for blood cancers.

Results from early phase clinical trials show that the technology significantly reduces the complications of transplantation and provides faster and better recovery for patients. With SCN support, ExCellThera is now expanding its reach into the United States and Europe.



EQ7

KEY FINDING: The Stem Cell Network is an effective and efficient model for supporting stem cell research and is deemed to have a fair and transparent governance and reporting structure. Early efforts on equity, diversity and inclusion have supported a n inclusive delivery model. Although alternative models of delivering government support to stem cell researchers are possible , the majority of interviewees noted they would not be as effective in supporting national -level research efforts.

Delivery of the Stem Cell Network

SCN operations costs as a percentage of total SCN expenditures ranged between 10.0% to 15.0% from fiscal years 2016-17 to 2019-20. For each fiscal year, this percentage was at or below the maximum 15% allotted towards ‘Operations and Office Administration’ from the Contribution Agreements with ISED.

	2016/17	2017/18	2018/19	2019/20
Stem Cell Network Operations	\$ 595,424	\$ 874,021	\$ 898,609	\$ 900,000
Outreach and Training	\$ 213,076	\$ 900,000	\$ 1,100,000	\$ 874, 761
Research Support	\$ 5,116,750	\$4,417,187	\$ 4,001,391	\$ 4,225,239
Operating as a % of Total Expenditures	10.0%	14.1%	15.0%	15.0%
Total SCN Expenditures	\$ 5,925,250	\$ 6,191,000	\$ 6,000,000	\$ 6,000,000

The majority of interviewees perceived SCN's performance, in terms of its design and how it delivers funding, to be working well. Potential considerations include:

- Providing more training for entrepreneurship and keeping close ties with successful companies which were seeded by the SCN in order to potentially have them give back to the stem cell community.
- Increasing the SCN’s visibility with industry to foster collaboration and leverage greater funding.
- Dedicating more resources towards regenerative medicine and stem cell therapy manufacturing and commercialization.

To what extent has the SCN been an efficient model for supporting stem cell research in Canada?

Are there alternative funding approaches that could support stem cell research?

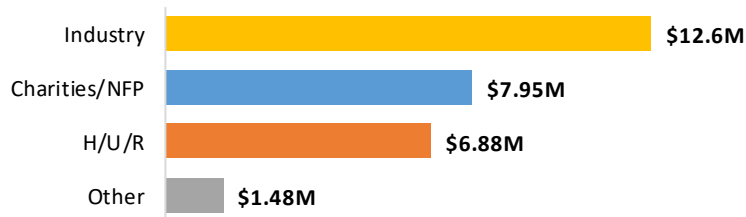


EQ7

Leveraging of ISED Contributions

The data review showed that, as a result of the SCN’s three multidisciplinary funding competitions, SCN collaborations leveraged \$28.9 million in partner funding from the \$19.8 million in ISED contributions committed to date. This indicates that for every \$1 in ISED contributions, ≈\$1.5 in partner funding was leveraged.

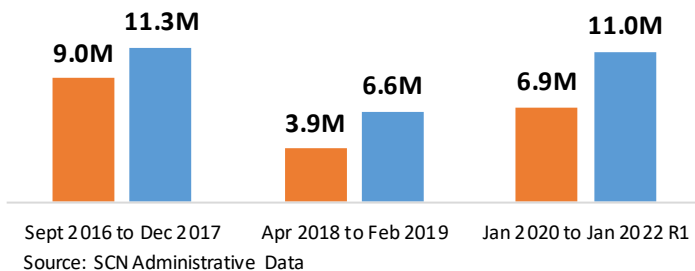
Total leveraged partner funding (\$28.9M) by partner source.



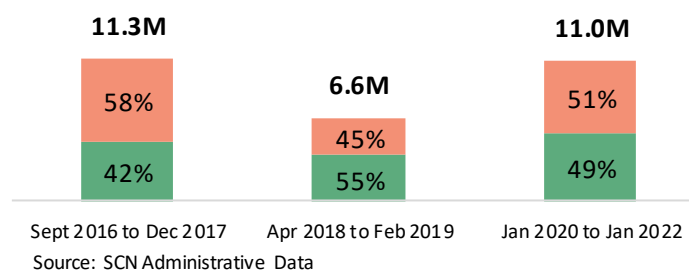
Source: SCN Administrative Data

Administrative data also substantiates that partner funding exceeded ISED contributions in each funding competition, consisting of a total of \$13.8 million in cash (48%) and \$15.1 million in-kind support (52%).

Partner funding leveraged per ISED contribution, by funding competition (\$).



Percentage of cash and in-kind support from partner funding, by funding competition (\$, %).



To what extent has the SCN been an efficient model for supporting stem cell research in Canada?

Are there alternative funding approaches that could support stem cell research?



EQ7

To what extent has the SCN been an efficient model for supporting stem cell research in Canada?

Are there alternative funding approaches that could support stem cell research?



Governance and Reporting

SCN governance and reporting is effective and well-structured.

- SCN governance was reviewed by a third party consultant in 2019, which led to the update of the SCN's by-laws, such as implementing proper term limits for Board members.

SCN governance was mentioned as being inclusive, having good and active leadership, and cognizant of areas requiring strengthening.

- Membership on the Board of Directors is updated regularly to ensure a diverse set of skills among Board members.
- Interviewees described the peer review structure of the SCN as robust and transparent. It is highly regarded, with multiple review committees involved before the Board ultimately makes a final decision.
- SCN governance suggestions included increasing scientist and private sector representation on the Board, and improving governance succession planning.

SCN researchers noted that the SCN has an effective and transparent reporting structure (via annual reports) and that the SCN minimizes paperwork and reporting requirements.

Equity, Diversity and Inclusion (EDI)

SCN officials and Board members remarked that EDI awareness is now part of the SCN's mandate. Board members are obligated to undertake EDI training sessions such as unconscious bias training to help reveal automatic patterns of thinking that may impede EDI practices. In addition, EDI-specific events have also become commonplace throughout Canada, including the Toronto Focus on Diversity lunch (70+ participants), and most notably at the 2019 Till and McCulloch meeting:

- Women in Regenerative Medicine Luncheon (70+ attendees);
- EDI in Research plenary presentation (250+ attendees); and
- Unbiased training for the SCN's Board of Directors (12 attendees).

The assessment process for research funding ensures that EDI issues are being addressed within projects, thereby improving inclusivity. The Research Management Committee is now asked to strategically look for diversity in the proposed research teams - it is an active requirement before making funding recommendations to the Board.

EQ7

To what extent has the SCN been an efficient model for supporting stem cell research in Canada?

Are there alternative funding approaches that could support stem cell research?



Global Funding Models

Global funding models are similar to the SCN in that government funding is provided to the respective stem cell and regenerative medicine organizations:

- **Australia:** Stem Cells Australia (SCA) was established in 2011 with the support of the Australian Government (A\$24 million in funding over 8 years). As part of the 2019-20 Budget, the Government allotted A\$150 million over 10 years to support stem cell research and deliver innovative, safe and effective treatments.
- **United States:** Since its inception in 2004 up to late 2019, the California Institute for Regenerative Medicine (CIRM) has awarded about US\$2.7 billion in grant funding. An additional US\$5.5 billion was allocated to the CIRM in 2020. The California Model offers a new paradigm for government funding of stem cell research and therapy development by authorizing the state to issue grants and/or loans (funded by bonds) over 10 years for all stem cell research (with a priority for embryonic stem cell) and other critical biomedical research opportunities.
- **UK:** The Biotechnology and Biological Sciences Research Council (BBSRC), the Engineering and Physical Sciences Research Council (EPSRC), and the Medical Research Council (MRC) fund the UK Regenerative Medicine Platform which was established in 2013, and is a £42 million initiative (£25 million from 2013-18 and £17 million from 2018-23) addressing the key translational challenges of regenerative medicine.

Alternative Stem Cell Funding Models

Almost all interviewees agreed that although alternative models (e.g., via an existing program) of delivering government support to stem cell researchers are possible, it would be difficult to improve upon the current model as no other existing mechanism (e.g., CIHR) currently targets a broad interdisciplinary cohort of researchers involved in stem cells like the SCN. Interviewees indicated that the SCN's network approach is the best way to fund stem cell research nationally, and to maintain and continue to build research capacity.

COVID-19 Response

- While outside the scope of the evaluation, SCN officials noted how quickly the SCN provided a response to support COVID-19 initiatives.
- The SCN provided \$0.68 million in funding towards three research projects (including the CIRCA-19 clinical trial) with partner support from the Ontario government, research institutions, industry and charities valued at \$2.3 million.
- This helps demonstrate the flexibility and efficiency of the current model.

Conclusions



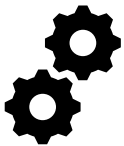
Relevance

There is a continued need for multidisciplinary and collaborative stem cell research due to its potential to lead to innovative therapies and treatments for chronic and degenerative diseases as well as the resulting economic benefits. The SCN is the only national organization in Canada with a sole focus on supporting stem cell research from beginning to end along with the training and career development of future stem cell researchers. However, the time-limited nature of ISED-SCN funding agreements can impact the SCN's ability to secure long-term funding from other sources.



Performance

The SCN has helped increase networking and collaboration among researchers domestically and internationally, particularly through the annual Till and McCulloch Meeting and the multidisciplinary requirements of SCN-funded projects. The network continues to expand the breadth and depth of knowledge related to stem cells, and the ethical, legal and social implications, by funding innovative research projects, supporting research publications in high impact journals, and sponsoring SCN researchers to present their findings at national and international workshops and conferences. This, in turn, enhances Canada's international standing and leadership in the stem cell and regenerative medicine fields. The SCN enables the career development and research opportunities of trainees, which contributes to a pipeline of qualified researchers that remain in Canada to pursue higher education and employment positions within all areas of Canada's stem cell and regenerative medicine sector. Lastly, through support and targeted funding for translational research, SCN-funded research has led to clinical trials, the development of patent applications, and the establishment and enhancement of start-up companies.



Efficiency

ISED funding has supported the SCN in leveraging partner funding and in developing a multidisciplinary and collaborative stem cell research network throughout Canada. The SCN is deemed to have a fair and transparent governance and reporting structure. Early efforts on equity, diversity and inclusion have supported an inclusive delivery model. Although alternative models of delivering government support to stem cell researchers are possible, the majority of interviewees noted they would not be as effective in supporting national-level research efforts.



Recommendations

The findings from the evaluation did not produce any recommendations.

Sources

¹ Global Regenerative Medicine Market Analysis & Forecast to 2025.

<https://www.researchandmarkets.com/reports/5144903/global-regenerative-medicine-market-analysis-and>

² Institute of Health Economics. 2020. Stem Cell/Regenerative Medicine in Canada: Current States and Future Prospects.

<https://ised-isde-gcdocs.ic.gc.ca/IC-GCDOCS/cs.exe?func=ll&objaction=overview&objid=25259585>

^{3,5} Decision Resources Group (DRG). 2017. Future Competitiveness of the Canadian Regenerative Medicine and Stem Cell Industry.

⁴ The Regenerative Medicine & Cell Therapy Sector in Canada. 2020. Commissioned by Innovation, Science and Economic Development Canada (ISED).

^{6,7} Bibliometric Analysis of Publication Results from Stem Cell Network Supported Research & Survey Results of International Stem Cell Experts. 2020. Report commissioned by the Stem Cell Network.

^{8,9} Training Tomorrow's Research Leaders: An impact analysis of the Stem Cell Network's Training Program.

<https://stemcellnetwork.ca/wp-content/uploads/2020/05/SCN-HQP-Report-English-FINAL.pdf>

Stem Cell Network Research Funding Programs

2016-17 to 2018-19

1) Clinical Trials	Supported phase I/II trials with the potential to be economically viable for healthcare systems and benefit patients.
2) Disease Team Program	Supported projects that developed novel cellular or stem cell-related therapeutic approaches to tissue repair and regeneration for specific diseases.
3) Impact Program	Supported translational, clinical translation, commercialization and public policy research.

2019-20 to Present

1) Advancing Clinical Trials	Focusing on novel cellular or stem cell-related therapeutic approaches to tissue repair and regeneration for specific diseases.
2) Fueling Biotechnology Partnerships	Supporting academic partnerships with emerging Canadian regenerative medicine biotech companies working to drive an innovative stem cell-based technology or therapy into the market/clinic.
3) Accelerating Clinical Translation	Supporting multidisciplinary research projects that are moving toward the clinic within five years, as well as translational research activities (including ELSI questions) associated with clinical trials.
4) Translation & Society	Supporting ELSI-led research that will address issues that impede the translation of innovative stem cell research.

Stem Cell Network Training Programs

Travel Awards Program: SCN supports HQP to learn and collaborate by enabling them to attend workshops and courses within Canada and internationally.

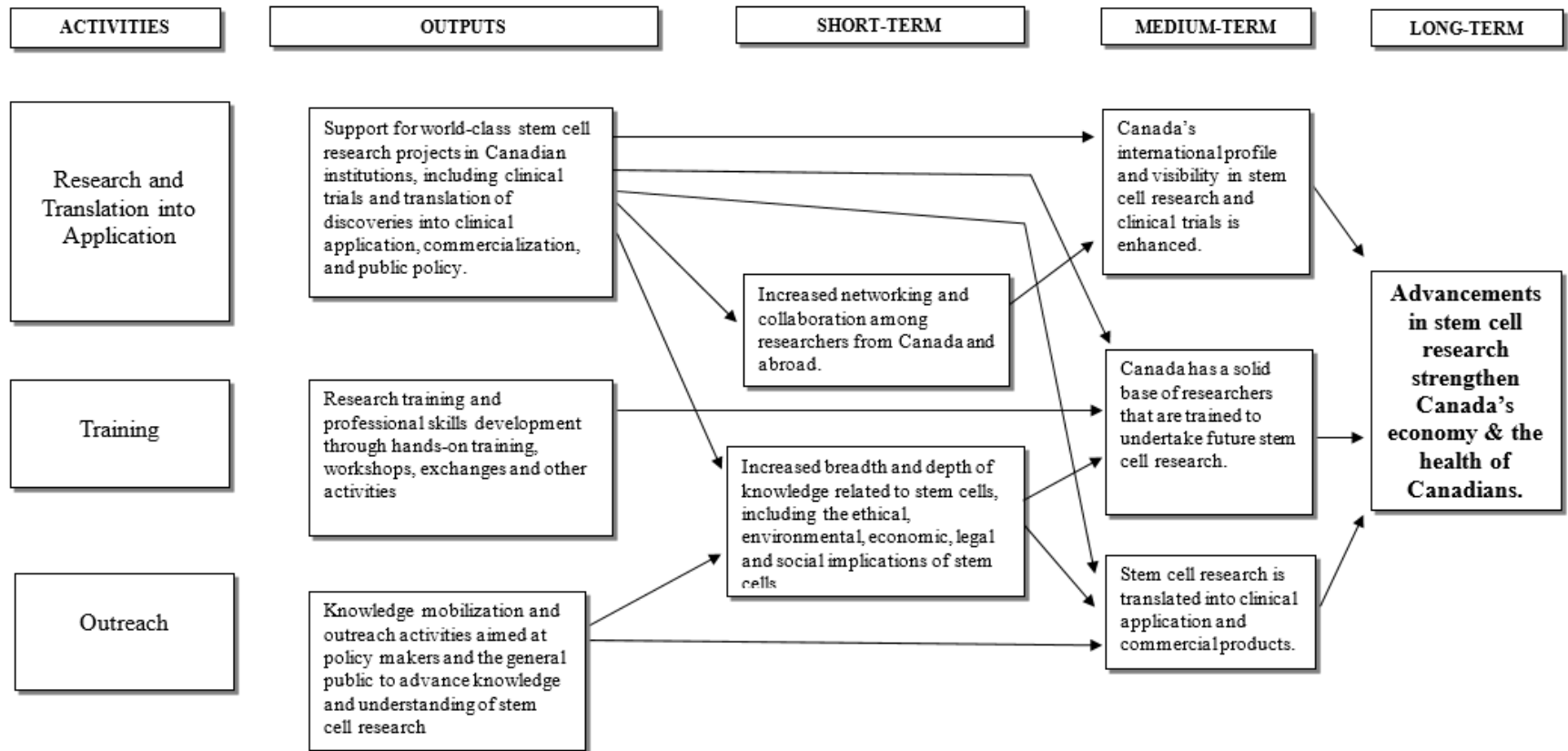
- Workshops supported by the SCN and partner organizations include, but are not limited to:
 - Current Trends in Biotherapeutics
 - RNA-Sequencing Analysis Workshop
 - UBC Flow Cytometry Course
 - Regulatory Steps Workshop
 - Scientific Communications Workshop
- The SCN has partnered with BioTalent Canada to offer access to a series of ‘Technical Skills Fundamentals’ online courses (e.g., Good Laboratory Practice (GLP), Good Manufacturing Practice (GMP), Good Clinical Practice, and others).
- The Training Program in Regenerative Medicine (TPRM) is a multi-disciplinary regenerative medicine graduate course, connecting participants with transplant, stem cell, and regenerative medicine researchers in Canada.

Exchange Program: Aims to provide researchers and trainees the opportunity to acquire specific skills or develop collaborations that will directly benefit their research project.

Stem Cell Network Outreach & Engagement Activities

- **Till & McCulloch Meeting (TMM)** is Canada’s largest annual stem cell and regenerative medicine conference, gathering stem cell scientists, clinicians, ethicists and policy-experts as well as industry representatives from Canada and abroad. The TMM showcases Canada’s place in the global stem cell ecosystem and provides attendees with opportunities to attend workshops, network, knowledge exchange, and hear about the latest scientific advancements.
- **StemCellTalks** is a nationwide initiative in which students learn about stem cell and regenerative medicine from scientists and experts in the field.
- **Stem Cells from the Sofa** is a virtual space to share scientific findings, insights and best practices related to stem cells and regenerative medicine. Presentations are broadcast live via webinar and include Q&A with the guest speaker.
- **Online Activity:** SCN ensures its online activities are both engaging and relevant, reflect the current best practices, and provide timely information on the latest scientific advancements, workshops, training opportunities.

Stem Cell Network Logic Model



Overview of 6 of the Stem Cell Network's 14 Clinical Trials

Burn wounds [SASS trial]

Health Issue: 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.

Clinical Trial: 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.
(Dr. Véronique Moulin at Université Laval)

Potential Treatment: 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.

Organ transplant rejection [ASCOTT]

Health Issue: Liver transplants save lives, but they also require recipients to take immune-suppressive drugs, to prevent the body from rejecting the foreign addition for the rest of their lives. These drugs mean greater susceptibility to a range of illnesses, including diabetes, cardiovascular disease, cancer, infection and kidney disease, which may result in a shorter overall lifespan.

Clinical Trial: Exploring how to use the patient's own stem cells to re-educate the immune systems to accept the new liver, making rejection unlikely and immune-suppressive medications unnecessary.
(Dr. Harold Atkins, Ottawa Hospital Research Institute, and Dr. Gary Levy, University Health Network)

Potential Treatment: If successful, it will revolutionize the field of solid organ transplantation and may even become a treatment that can reduce the need for such transplantation.

COVID-19 [CIRCA-19]

Health Issue: As the COVID-19 pandemic continues to grip the world, uncertainty about the future persists for Canadians. Canadian scientists are fully engaged in addressing COVID-19 by developing an innovative cell-based treatment.

Clinical Trial: Administer a specialized cell product for critically ill patients, with the goal of tempering down the aggressive inflammatory response that prevents the transfer of oxygen to the blood and damages the lungs.
(Dr. Duncan Stewart from the Ottawa Hospital Research Institute)

Potential Treatment: Recent research demonstrated that of seven patients given a similar cell product, lung function and symptoms improved significantly after 48 hours.

Septic shock [CISS]

Health Issue: Each year in Canada, 100,000 patients experience septic shock, a condition that is fatal in 30-40% of cases. This severe infection accounts for half of all critical care costs (\$4B per year) and survivors face long-term recovery with the possibility of permanent disability.

Clinical Trial: Testing a potential therapy that uses mesenchymal stem cells (MSCs) to tell the immune system to calm down and let repairs begin.
(Dr. Lauralyn McIntyre Ottawa Hospital Research Institute)

Potential Treatment: If successful, the therapy would save thousands of lives, improved quality of life for survivors and result in significant health care savings.

Heart disease [ENACT-AMI]

Health Issue: not provided.

Clinical Trial: Determining whether early-outgrowth autologous endothelial progenitor cells (EPCs) can improve cardiac repair after a large myocardial infarction, or heart attack, and establish whether adding extra copies of a particular gene will enhance the regenerative activity of a patient's own EPCs by overcoming the deleterious effects of older age and other cardiac risk factors.

(Dr. Duncan Stewart from the Ottawa Hospital Research Institute)

Potential Treatment: If successful, this therapy could transform the management of patients who suffer debilitating cardiac damage after a heart attack and prevent the progression to heart failure with the attendant devastating consequences on quality of life, survival and costs to the health care system.

Eye repair [CECA]

Health Issue: Unilateral corneal limbal stem cell deficiency (LSCD) is a severe eye disease caused by damage or depletion of the corneal stem cells in the eye following trauma or disease. Once the corneal stem cells are destroyed, the epithelial tissue on the surface of the cornea can no longer regenerate or heal spontaneously, resulting in chronic inflammation and invasion by the conjunctiva and its blood vessels, resulting in a scarred and cloudy cornea and even vision loss in many patients.

Clinical Trial: Test methods to reconstruct skin and eye tissues, and implement a new cell therapy that will be accessible to Canadian ophthalmologists.
(Dr. Lucie Germain at Laval University)

Potential Treatment: Improved corneal health and vision for patients suffering from LSCD.

Examples of Companies Created or Enhanced with SCN Funding



Aspect Biosystems is a privately held biotechnology company, located in Vancouver. Led by co-founder and CEO Tamer Mohamed, it operates at the leading edge of 3D bioprinting and tissue engineering. Aspect's proprietary Lab-on-a-Printer™ platform technology is enabling advances in the understanding of fundamental biology, disease research, the 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.



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 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. ExCellThera's platform combines this molecule with an optimized culture system that allows the rapid preparation of the therapeutic cells. Results from early phase clinical trials show that the technology significantly reduces the complications of transplantation and provides faster and better recovery for patients. With SCN support, ExCellThera has emerged as a Canadian commercialization success and is now expanding its reach into the United States and Europe.



StemAxon, founded by SCN-supported investigator Dr. Gilbert Bernier, is pursuing breakthroughs in both neurodegenerative diseases such as Alzheimer's, and retinal diseases. 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.

Annex E Cont'd



Morphocell Technologies was a company founded by Drs. 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 company is seeking to initiate human clinical trials within the next two years.



Satellos was founded by Dr. Michael Rudnicki with a platform to regenerate muscle tissue more effectively. 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. The Satellos pharmacologic approach restores this balancing act for greater muscle repair and healing. 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 for a range of neuromuscular diseases.