



## 2017 Pre-Budget Submission to the House of Commons

### Standing Committee on Finance

#### **Introduction**

Regenerative medicine is one of the biggest frontiers of our time. The opportunities for improvements to people's lives are extraordinary. The potential economic and health benefits are remarkable. Through leading edge research, novel cell therapies, precision medicine and development of new technologies, regenerative medicine based on stem cell research is starting to provide significant economic value and disrupt the delivery of health care. Today, we are at the tipping point; just a few decades ago, the focus was on discovery. Now, world class clinical trials are taking place and producing innovative treatments that have the potential to change lives and provide hope to those suffering from debilitating illness. Canada has an opportunity now to recognize regenerative medicine as the key to unlocking massive benefits to society – personalized health benefits, system benefits, and economic benefits.

The Standing Committee on Finance is soliciting policy advice from stakeholders in advance of Budget 2018, with a specific eye on productivity – making Canadians more productive, and making Canadian businesses more productive.

**By helping foster a framework that maximizes the potential of regenerative medicine to develop and thrive, policymakers can help catalyze three important productivity-enhancing outcomes:**

- ✓ **Reducing the economic productivity loss of chronic/episodic illness sufferers not engaging fully in the workplace and economy;**
- ✓ **Increasing the efficiency and output of our health care system to deliver superior outcomes to patients while slashing costs of traditional, conventional, long-term treatments;**
- ✓ **Improving the ability of high-value knowledge economy research to proliferate high-value knowledge economy bio-tech businesses – helping Canadian start-ups in a sector that will dominate the future of health care and bio-science.**

## **Background – the Science**

Regenerative Medicine is described as the process of replacing, engineering or regenerating human cells, tissues or organs to establish or restore normal function. The key driver in regenerative medicine is stem cell science – an area of research pioneered in Canada. Stem cell science is just as Canadian an innovation as the discovery of insulin or the development of the Canadarm. It is not only iconic, but a major contributor to the global scientific enterprise, while bolstering Canada’s position as a health innovation leader.

Stem cells are unique due to their capacity to develop into any cell and repair any damaged and diseased tissue or organ in the human body. These cells and the broader regenerative medicine field hold enormous potential for societal benefit, from both health and economic perspectives. From a health standpoint, they have the power to dramatically change and even cure diseases that afflict a large proportion of the world’s population. As the science continues to mature, it is reasonable to believe that curative therapies are around the corner for diseases such as multiple sclerosis (MS) and diabetes, and for life threatening conditions such as septic shock.

### **The Potential**

Stem cell therapies are already being used to treat leukemia, multiple myeloma and other blood cancers. They hold a tremendous potential to do even more, including treating people suffering from respiratory diseases, heart disease, other cancers, ocular disease, diabetes, spinal cord injury, MS, Crohn’s disease, auto-immune disorders, Parkinson’s and to reverse tumour growth.

Stem cells were discovered in this country over 50 years ago by two Canadian scientists. Canadian researchers have continued to lead, having published 8,342 stem cell-related research articles between 2000 & 2014 alone. The impact of Canadian articles, as measured by citations, is high.<sup>1</sup>

**However, advances will only be made if the innovative research currently taking place in labs across the country is able to advance. This requires a stable and predictable funding environment that will support goal-directed research at all stages, including early translation, pre-clinical & clinical, proof of principle, pre-commercialization & commercialization, ethical, legal and social research. It will also allow Canada to bring its scientific muscle to international research and clinical trial collaborations.**

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<sup>1</sup> CCA, 2017 Building on Canada’s Strengths in Regenerative Medicine

### Examples of Canadian Scientific Excellence in Regenerative Medicine

- **Dr. Tim Kieffer from UBC**, developed a protocol that can turn stem cells into insulin producing cells in a matter of weeks. As a result, we are one step closer to having an unlimited supply of insulin-producing cells to treat patients with Type 1 diabetes. Dr. Kieffer's research long supported by SCN and the Canadian Diabetes Association, is now moving towards clinical trials and his work offers hope for thousands in Canada and around the world.
- **Dr. Freda Miller from the University of Toronto**, discovered that skin is a viable source of stem cells. This ground-breaking discovery opened the door to learning how to treat injured nervous systems, more specifically spinal cord injuries. Today, Dr. Miller and her team are conducting a promising clinical trial using the diabetes drug Metformin to stimulate brain regeneration in children who have undergone radiation for treatment of a malignant brain tumor. The results are very promising and offer hope for timely clinical application.
- **Drs. Harry Atkins and Mark Freedman from the Ottawa Hospital Research Institute** had their major breakthrough for treating early, aggressive forms of MS published in *The Lancet*. The procedure uses stem cells and chemotherapy to eradicate MS in a small subset of patients. This paradigm shifting approach is anticipated to become the new standard of care internationally, and with more research could benefit a larger population of MS patients.
- **Dr. Denis Claude Roy and colleagues from the Hopital Maisonneuve-Rosemont, Université de Montréal** have developed a novel strategy that enables stem cell transplantation from partially matched adult donors. This Canadian discovery virtually eliminates rejection without the use of immune suppressors. It is now being evaluated in a Canada led Phase III clinical trial performed internationally.

#### **Background – the Business Translation**

Canada is a leader in this science, but we face challenges in commercializing therapies, and in spinning off viable biotech companies and ensuring they remain anchored in Canada.

The number of Canadian biotech firms within the regenerative medicine space is small but growing. Other jurisdictions such as California, Japan and Great Britain are doing this better and more comprehensively than we are. Consequently, a Canadian-invented science is falling behind the competition in turning research into patents and companies. The good news is that RMAC members recognize the challenge and are focused on translating discovery into commercial reality for the benefit of Canada.

In Canada, 3,000 patents are required before one spin off is created. In a recent study of six countries with a competence in regenerative medicine, Canada placed fifth in translating science into start-ups.

The potential is limitless, given the right conditions and resources to invest adequately in research, and the commercialization and availability of stem cell based therapies. The countries and enterprises that establish a leading position in new stem cell therapies will have advantages for their populations suffering from related illnesses, and for their health care systems overall. Similarly, the patent holders and manufacturers involved in stem cell therapies will establish global markets for their life-changing and life-saving therapies.

With the right strategy that supports Canadian researchers, entrepreneurs and up-and-coming SMEs Canada can and will capture a significant piece of the global regenerative medicine market – market that continues to grow and is valued to be an estimated \$49B by 2021.<sup>1</sup>

There are clear and demonstrable reasons for Canada stands to be at the forefront of stem cell research and technology.

### **The Missing Policy Links**

Throughout the regenerative medicine ecosystem, we hear a common message about missing links in the pipeline from discovery research to intellectual property to full commercialization of therapies and establishment of biotech companies:

- **Insufficient access to venture capital and seed funding:** Many venture capital firms will only invest in less risky late stage validated opportunities, leaving little financing available to develop therapeutics in the early stages.
- **Weak receptor capacity for regenerative medicine therapies and products:** Health care systems are driven to deliver therapies at the lowest possible cost, rather than supporting therapies that support economic growth and better long-term health outcomes. Consequently, industry looks outside of Canada for its customer base where access and adoption are more easily facilitated.
- **Strengthening Canadian infrastructure:** To compete on the global stage, maximizing output from existing GMP facilities<sup>2</sup>, as well as investing in new, leading-edge biomanufacturing centres that specialize in cell and viral vector manufacturing is critical. A strategic focus in this area will generate both direct and indirect economic benefits for Canada. To be successful leading-edge equipment and technologies, along with a highly skilled, Canadian trained labour force is needed. Investment, through the Innovation Superclusters Initiative and/or the Strategic Innovation Fund will allow Canada to effectively compete on the world stage, retain IP and create high quality jobs.
- **Regulatory environment and clinical trials:** The regulatory burden faced by researchers and businesses is cumbersome – particularly relative to other leading regenerative medicine

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<sup>2</sup> GMP stands for Good Manufacturing Practice. Canada has seven GMP manufacturing facilities that produce high quality cells for pre-clinical studies or clinical trials. These centres are critical for the delivery of innovative cell therapies that will treat chronic disease and illness.

jurisdictions. A 2012 parliamentary report that studied Canada’s clinical trials environment noted that our “deficiency results in companies having to submit multiple research proposals to meet the requirements of research ethics boards at numerous trial sites.

Thus, considerably more time and effort is needed to get clinical trials designed, approved and started in this country. As a result, the cost of testing drugs and biologics in clinical trials is high in Canada, making it less desirable to the industry as a destination for clinical trials.”<sup>3</sup>

- **Access to Talent:** Canada is known for the strength of its university system and the quality of its graduates. RMAC organizations partner together each year to provide leading edge training programs and workshops on scientific techniques vital for stem cell research and clinical therapies. Today, training is also offered on commercialization topics such as the business of regenerative medicine, clinical translation, science communication and entrepreneurship. More can and should be done, including partnerships, internships, recruitment incentives and facilitation of global top talent.

### **The Opportunities for Health Care Savings and Economic Productivity**

The opportunities in regenerative medicine lie in saving Canadian lives, potentially eliminating entire varieties of chronic or episodic illness, and in long-term savings to the health care system in therapy, and savings to the economy in mitigating productivity loss.

Chronic disease consumes 67% of all direct health care costs, and cost the Canadian economy \$190 billion annually – \$68 billion is attributed to treatment and the remainder to lost productivity.<sup>1</sup>

### **Conclusion**

The regenerative medicine community has long been known for its strong commitment to collaboration. It is envisioned that industry, academia, government and RMAC organizations that support the knowledge mobilization chain will work as partners to realize the development of a thriving Canadian regenerative medicine sector that will generate both health and economic benefits for Canadians.

Canada is now at a tipping point where we are seeing the disruptive nature of stem cells leading to transformative therapies. Canada has some of the best scientific minds in the world based in universities, research institutes and hospitals across Canada. These researchers are situated across the country, and supported by federal organizations such as SCN, CCRM, CellCAN and CDRD. Combined, these groups provide the research, knowledge mobilization, clinical and commercialization support needed to move discoveries from the *bench to bedside*. Hubs of strength in regenerative medicine exist

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<sup>3</sup> Senate Standing committee on Social Affairs, Science and Technology. 2012  
<https://sencanada.ca/content/sen/committee/411/soci/dpk/01nov12/execsum-e.htm>

in Vancouver, Calgary, Toronto, Ottawa, Montreal and Quebec City. Researchers who work on the policy, legal and ethical questions relevant to stem cells are based in Alberta, Montreal and Nova Scotia.

Regenerative medicine has the potential to lead to innovative technologies and treatments that will improve the health of those suffer from disease and illness, while also contributing to the economy through tax revenue, jobs and company growth. Collectively, sector leaders, with support from government partners, have built a competitive advantage for Canada – an advantage we now have the opportunity to leverage and fully realize. However, moving forward decisively is imperative if Canada is to emerge as a global leader.

It is important that Canada build on its strong foundation in this field, but also rebalance its policy framework to support the entire pipeline, from basic science right through to commercialization.

Regenerative medicine, and the stem cell research that underpins it, is a strategic area of investment; as such, government should continue to support this area, and to approach it in a more strategic and system-based manner.

**About the Regenerative Alliance of Canada (RMAC)**

*RMAC is a voluntary organization comprised of national, provincial and regional organizations that are committed to building a robust regenerative medicine sector in Canada. All members have mandates relevant to stem cell research and/or regenerative medicine. The founding members include: Centre for Commercialization of Regenerative Medicine (CCRM); The Centre for Drug Research and Development(CDRD); CellCan; Medicine by Design; Ontario Institute for Regenerative Medicine (OIRM); ThéCell; Canadian Stem Cell Foundation, and Stem Cell Network (SCN).*