

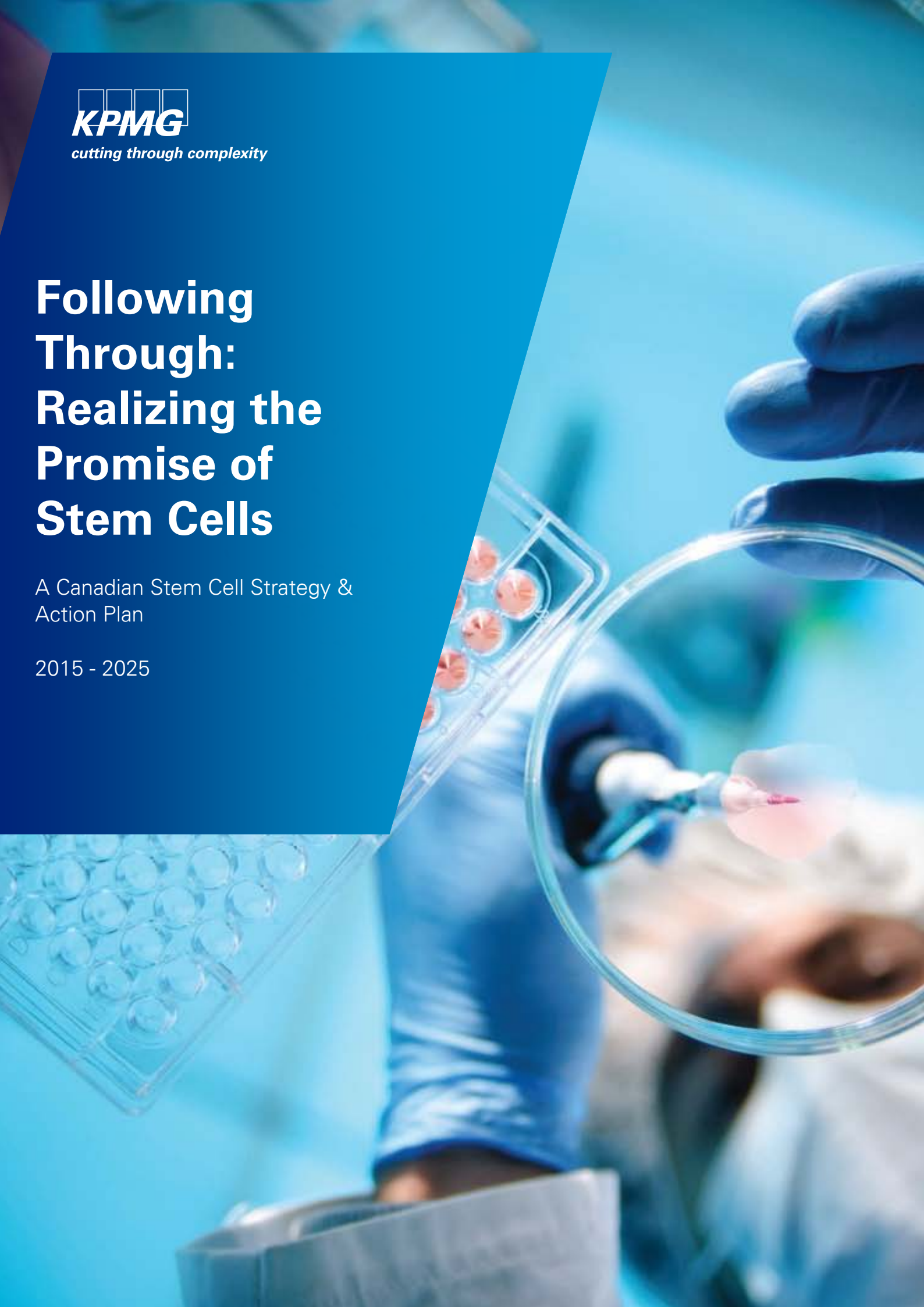


*cutting through complexity*

# Following Through: Realizing the Promise of Stem Cells

A Canadian Stem Cell Strategy &  
Action Plan

2015 - 2025



## Notice to Reader

This document has been prepared by KPMG LLP (“KPMG”) for the Canadian Stem Cell Foundation (“Client”) on behalf of the Joint Strategy Working Group for the Canadian Stem Cell Strategy, composed of representatives from the Canadian Stem Cell Foundation, the Centre for Commercialization of Regenerative Medicine, the Stem Cell Network, the Health Charities Coalition of Canada, the Ontario Stem Cell Initiative, the CellCAN Regenerative Medicine and Cell Therapy Network and the Ontario Bioscience Innovation Organization pursuant to the terms of our engagement agreement with the Client dated October 31, 2013 (the “Engagement Agreement”). KPMG neither warrants nor represents that the information contained in this document is accurate, complete, sufficient or appropriate for use by any person or entity other than the Client or for any purpose other than set out in the Engagement Agreement. This document may not be relied upon by any person or entity other than Client, and KPMG hereby expressly disclaims any and all responsibility or liability to any person or entity other than Client in connection with their use of this document.

August, 2014

# The 10-Year Action Plan at a Glance

## The Advanced Cell Therapy and Regenerative Medicine Opportunity for Canada

Advanced cell therapy and regenerative medicine is the next frontier in the development of innovative therapeutics and treatments. This transformation in health care represents a huge opportunity in treating and curing disease, improving the sustainability of health care and creating new high-value skilled jobs and economic opportunity for Canada.

This new field includes stem cell-based technologies, cell therapy and regenerative medicine. Together they are changing how we treat patients and deliver health care. Stem cell technologies have revolutionized the discovery of new drugs and enabled faster and safer drug testing, thus accelerating the process of drug development. Cell therapy uses living cells as treatments. Its potential to cure or transform serious medical conditions lies in the nature of cells and their ability to interact with the body at levels of complexity many orders of magnitude greater than conventional drugs. Regenerative medicine is focused on the regeneration of tissues and organs using all the different therapeutic platform technologies available: small molecule drugs, biologics, medical devices and cells.

Stem cell technologies, cell therapy and regenerative medicine are closely entwined and will ultimately transform the practice of medicine from today's model of continual interventions to single treatment cures.

***“Stem cell technologies, cell therapy and regenerative medicine are closely entwined and will ultimately transform the practice of medicine.”***

From a health standpoint, they have the power to dramatically redefine quality of life by altering the disease trajectory and curing chronic and degenerative conditions that afflict a large proportion of the world's population.

Finding new treatments for prevalent and highly burdensome conditions such as diabetes or cardiovascular disease is also an economically exciting prospect. The ability of cell therapies to provide a curative option for incurable chronic diseases represents a huge asset in the worldwide effort to contain increasing health care and medication costs. Consequently, nations that discover and develop these breakthrough technologies will not only reduce the burden of disease locally, but also globally.

Recognizing the potential power of stem cells and the exciting economic benefits they may bring, jurisdictions such as California, Japan, and the UK have started to invest significantly in this sector. As yet, there is no clear global leader in the cell therapy industry. This, however, is set to change given the escalating financial and policy commitments made by these governments over the last few years.

## A Canadian strength

Canada is a world leader in this field. The existence of adult stem cells was first demonstrated by Canadian scientists Drs. James Till and Ernest McCulloch in the early 1960s. They paved the way for subsequent generations of Canadian scientists who continue the legacy as world leaders in stem cell research.

Canada's leadership in stem cell discoveries and our collaborative, multidisciplinary and cohesive research community provide a significant advantage in this global race for improved health and prosperity. Strategic investments in research networks, organizations and infrastructure, such as the Stem Cell Network (SCN), the Centre for Commercialization of Regenerative Medicine (CCRM) and the newly funded CellCAN Regenerative Medicine and Cell Therapy Network have positioned Canada to lead in the next wave.

Canada can seize this opportunity by building on our wealth of research and network infrastructure, and consolidating leading technologies into novel therapies to create a vibrant cell therapy and regenerative medicine industry and drive health care change. To achieve this, Canada must continue

to invest, but do so strategically to compete successfully on a global scale and generate a return on our investment in discovery research.

**The Canadian Stem Cell Strategy & Action Plan in a nutshell**

**“‘Made in Canada’ could be the hallmark of cell therapies in much the same way as ‘Made in Switzerland’ is for quality timepieces.”**

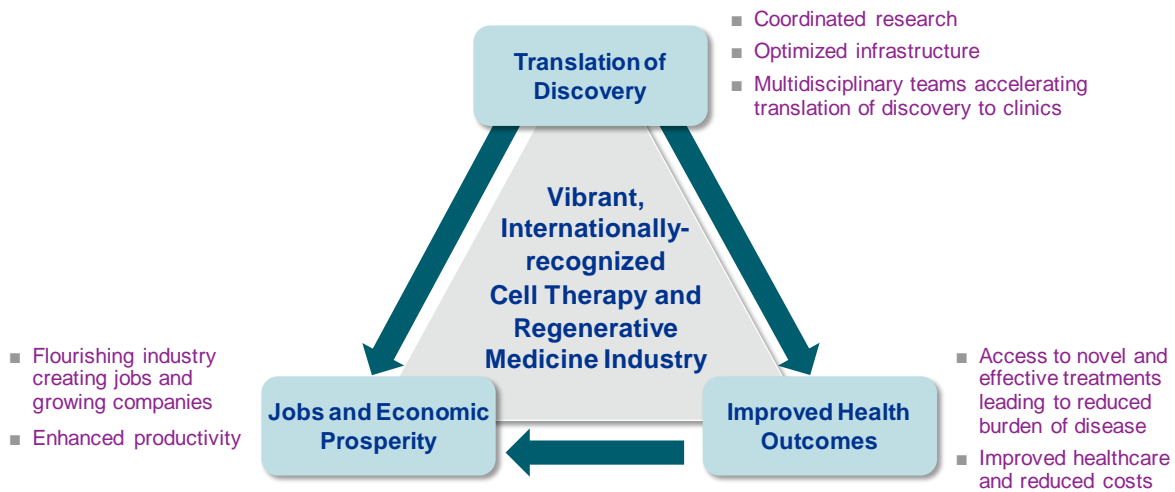
The proposed Action Plan is a 10-year transformative initiative to create a successful, internationally recognized cell therapy and regenerative medicine industry that will translate discovery research, wherever created, into new technologies, therapies and products. It will improve health outcomes for Canadians and create

new skilled jobs and economic prosperity. Canada will emerge as a leading global cell therapy nation complete with its own manufacturing base, ensuring that the nation’s industry is securely embedded, globally competitive and sustainable in the long term. Manufacturing is the key to success in capturing the real value of the future multibillion-dollar market for cell therapies. “Made in Canada” could be the hallmark of cell therapies in much the same way as “Made in Switzerland” is for quality timepieces.

Desired outcomes by 2025

The Action Plan will drive the research and development (R&D) of the closely interconnected triad of stem cell technologies, cell therapy and regenerative medicine through to global commercialization and clinical application towards the three overarching outcomes and specific sub-outcomes described in Figure 1 (see Section 4.2 for further details).

Figure 1: Outcomes of the Canadian Strategy & Action Plan



The Strategy and Action Plan addresses key gaps, drives strategic coordination of efforts and integrates assets across the country. The goal is to produce five to 10 therapies within 10 years that can help transform the health care landscape. The potential benefits are illustrated in the following examples:

- *Development of a Novel Cell Therapy for Diabetes:* The number of Canadians living with diabetes is expected to reach 4.8 million in 2014 (Canadian Diabetes Association, 2014). Diabetes consumes 3.5% of Canadian public health care spending. Its direct health care and indirect economic costs are expected to surpass \$16 billion by 2020 (Canadian Diabetes Association, 2009) The transformational potential of stem cells to cure diabetes by restoring insulin production could mitigate a significant share of health care expenditures related to complications, long-term

disabilities, and premature mortality as well as bring significant indirect economic benefits not only to patients but society as a whole.

- *Development of Novel Treatments for Cardiovascular Disease:* Every seven minutes, a Canadian dies from heart disease or stroke (Statistics Canada, 2011). As the leading causes of deaths in Canada, heart disease and stroke cost the economy \$20.9 billion every year in physician services, hospital expenses, and lost productivity (Conference Board of Canada, 2010). Exciting clinical trials are already underway to explore stem cells' potential to regenerate damaged or scarred heart tissue. The implications of success are tremendous and highly transformative for global health care and could relieve a significant burden on the broader economy.

***“Canadian companies have the aptitude to compete with the world’s best.”***

These two examples provide a glimpse into the benefits that cell therapies could bring.

Economic analysis from CCRM estimates that a fully funded stem cell strategy could generate in

excess of 12,000 jobs for Canadians via the growth of existing companies and the development of new enterprises marketing products globally. The related supply chain would anchor the industry in the country and attract outside companies to join the cluster, helping to create a major competitive advantage over other locations. Recent Canadian successes such as STEMCELL Technologies, a home grown company with over 500 employees that sells over 1,500 products to almost 80 countries, demonstrate that Canadian companies have the aptitude to compete with the world’s best (see page 23 of Section 3.3.1 for a case study on STEMCELL Technologies).

### Five Strategic Pillars and Key Enablers

The Action Plan focuses on five strategic pillars and associated initiatives that will develop new breakthrough therapies for Canadians and the rest of the world and build a successful and internationally recognized multibillion-dollar industry in Canada that is all-encompassing, sustainable and scalable to support future market demands:

- **Multidisciplinary science** – Embrace, leverage and optimize our uniquely multidisciplinary foundation of assets, expertise and networks across the country to maximize translation of discovery and accelerate access to novel therapies for patients.
- **Market-driven commercialization** – Create and grow a vibrant stem cell/regenerative medicine ecosystem to support company creation and sustainability by increasing access to capital, enabling platform technologies and other commercialization services, and building advanced manufacturing capacity, which together will attract international investment.
- **Advanced cell manufacturing** – Build on Canada’s strong foundation of bioengineering expertise and cell processing infrastructure to emerge as a global cell manufacturing leader with capacity across the full spectrum of manufacturing from process development, to small-scale clinical and large-scale commercial manufacturing.
- **Regulatory enablement** – Catalyze the development and growth of a cell therapy industry to improve health outcomes and increase prosperity for Canadians.
- **Clinical trials leadership** – Bolster our clinical trial capabilities and infrastructure through better coordination and strategic funding to become the preferred global clinical trial destination.

These strategic pillars capitalize on our strengths and address key gaps in the current ecosystem. Successful delivery across each of the strategic pillars depends on the following key enablers: a financing framework to enable public and private investment; a robust recruitment and training pipeline to attract, develop and retain talent; national coordination and optimization of Canadian assets and infrastructure; strategic international partnerships and branding; and a strong partnership with Canadians.

### Priorities for Action

The following priority actions will drive the implementation of the Canadian Stem Cell Strategy & Action Plan and initiate the transformation of the Canadian cell therapy and regenerative medicine industry:

1. **Multidisciplinary science:** Support and maintain a strong, vibrant multidisciplinary R&D pipeline and training network – building off a sound base of discovery research funded through granting councils and agencies and leveraging successful Networks of Centres of Excellence (NCE) initiatives.
2. **Capital mobilization, company creation and technology development:** Seed and support private sector-led initiatives with investors to mobilize private capital and expertise to create and grow innovative companies that can develop and commercialize new technologies therapies and products. Enhance existing technology development programs with industry.
3. **Regulatory enablement:** Implement a streamlined regulatory and reimbursement pathway for advanced cell therapies that will: be an international benchmark for safety and efficacy; increase access to new therapies for patients; and be clear, transparent and predictable for industry and congruent with other leading jurisdictions.
4. **Clinical trials and advanced manufacturing:** Establish a clinical trials co-development fund to support and attract early stage clinical trials in stem cells/regenerative medicine that will position Canada as an international destination/partner in clinical development. Lead and support strategic initiatives in advanced cell manufacturing that will establish long-term manufacturing capability and attract investment.
5. **Reimbursement:** Explore and develop recommendations and options for a potential reimbursement framework for advanced cell therapies that builds on protocols established by the Canadian Agency for Drugs and Technologies in Health and the Common Drug Review.

### Leadership and Funding

To realize the promise of stem cells and regenerative medicine and to position Canada as global industrial leader in the future multi-billion dollar market, the Canadian Stem Cell Strategy & Action Plan will require a public-private investment in the order of \$1.5 billion over 10 years (\$500 million federal commitment and \$1 billion from other sources). Success depends on both public and private sector leadership and investment. Government plays a critical role in endorsing and seeding support, while the private sector, including industry, philanthropy, foundations, health charities and the finance and investment community must drive the strategic priorities and actions to achieve the vision of global leadership to create economic prosperity for Canada and improve health outcomes for all.

### What will Success Look Like in 2025? – An Economic Snapshot

A flourishing cell therapy and regenerative medicine industry will stimulate economic growth directly and indirectly in the short as well as long term. The cell therapy and regenerative medicine industry’s dependency on other supporting sectors, such as transport and logistics, will **indirectly** promote growth by increasing business transactions (revenue) and driving demand (job creation). Under strong leadership in the sector and with the appropriate strategic initiatives in place, the **direct** outcomes will include (but are not limited to) the following:

Table 1: Economic benefits of investment in the Canadian stem cell industry

	Short-term Benefits	Long-Term Benefits
Attraction of foreign risk-capital investment	✓	✓
Building and maintenance of infrastructure	✓	✓
Knowledge economy talent	✓	✓
Job creation (direct and indirect)	✓	✓
Company creation and/or relocation	✓	✓
Development of advanced manufacturing capacity		✓
Establishment of strategic alliances/ partnerships	✓	✓

Based on some key assumptions built on prospective company creation and clinical trials data, CCRM economic analysis predicts the growth of the cell therapy and regenerative medicine industry. The chart below compares a number of key economic output statistics in 2015 with those cumulatively created over the following 10-year period.

Table 2: Projected economic output of Canada's stem cell industry between 2015 and 2025

	2015	Cumulative: 2016 to 2025
Companies created	2	20
Tax revenue	\$2 million	\$405 million
Number of clinical trials	40	430
Expenditure on clinical trials	\$90 million	\$1,390 million
Number of marketed products	0	9
Total jobs created in Canada	180	12,080

Source: CCRM Economic Analysis of Canadian Stem Cell Industry - March, 2014





**Letter of Support**  
**Dated: August 12, 2014**

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Dr. Julia Levy  
Mr. Geoff MacKay


Dr. Christopher Mason  
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Dr. Stephen Minger  
Dr. Martin Pera

As a member of the International Expert Advisory Panel for the Canadian Stem Cell Strategy, I have reviewed drafts of the document entitled ***Following Through: Realizing the Promise of Stem Cells – A Canadian Stem Cell Strategy & Action Plan 2015-2025***. I support and endorse the final version of this document.



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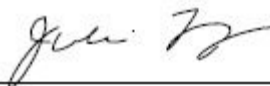
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