CellCAN

Regenerative Medicine and Cell Therapy Network

GOING FURTHER – TOGETHER

Strategic Plan 2015-2020

October 22nd, 2015

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The Strategic Planning task force members, listed below, have played a key role in leading this effort and in articulating CellCAN's mission and vision into a strategic plan for the next five years.

- Dr Pierre Duplessis, Chair of the Strategic Planning task force, Chair of the Board of Directors
- Dr. Armand Keating, Chair of CellCAN Steering Committee, member of the Board of Directors
- Dr. Denis Claude Roy, CEO of CellCAN

We also wish to thank for their time, feedback and vision the members of the Steering Committee, which regroups representatives of all CellCAN's network affiliates.

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FOREWORD

In Canada's global efforts to be the lead of the next wave of development in medicine - stem cell and regenerative medicine – CellCAN got funded in 2014 to harmonize efforts through knowledge sharing across facilities in order to produce high quality cell therapies, accelerate the implementation of novel cell therapy, thus proactively engage with stakeholders. It was also to promote the implementation of innovative cell therapies by disseminating knowledge to clinical centers, industrial partners, as well as to patients and the public at large.

In so doing, CellCAN will become the rally point for all stakeholders in the domain of stem cell and regenerative medicine.

This plan aims at setting the course of action for this vision to become reality. It reaffirms CellCAN' mission and provides the organization with a clear course of action over the next 4 years. It's activities address stem cell and regenerative medicine stakeholders, i.e. all those having an influence or being influenced by CellCAN: from the general public, to patients, health care professionals, scientists, policy makers, potential investors, Canadian and provincial governments, research facilities and partners.

Vision, mission, goals and objectives in this document are drawn from in depth consultative process and represent a large consensus. The way ahead will be a fascinating journey and we are confident to achieve the proposed deliverables with success.

1 CONTEXT – WHAT IS CELLCAN

1.1 Description

CellCAN brings together the main Cell and Tissue manufacturing facilities in Canada. These facilities allow for clinical trials at the forefront of stem cell transplantation, tissue repair and immunotherapy. CellCAN synergizes the resources and knowhow of these facilities, and promotes the development of new processes in cell-based therapy.

Incorporated as an independent non-profit corporation in February 2014, CellCAN was granted a $3 million budget over 4 years including:

- $1.6 million from the Networks of Centres of Excellence (NCE) – knowledge mobilization program
- $400 000 from the Hôpital Maisonneuve-Rosemont Foundation
- $1 million as "in kind" contribution from various organizations across Canada

1.2 Vision

Our vision is that CellCAN will be the key enabler of making cell-based therapy the next pillar[1] of 21st century medicine in Canada.

1.3 Current mission

CellCAN's mission is to mobilize knowledge and stakeholders across Canada to significantly advance regenerative medicine and cell therapy research and clinical development.

1.4 Founding CellCAN Network Affiliates

- Centre of Excellence in Cell Therapy (Maisonneuve-Rosemont Hospital)
- Orsino Cell Therapy Translational Research Laboratory (Princess Margaret Hospital)
- Ottawa Hospital Research Institute (University of Ottawa)
- Centre Multidisciplinaire de Développement du Génie Tissulaire (Université Laval)
- Alberta Cell Therapy Manufacturing (University of Alberta)
- Centre of Genomics and Policy (McGill University)
- Michael Smith Laboratories (University of British Columbia)

1.5 Governance and management

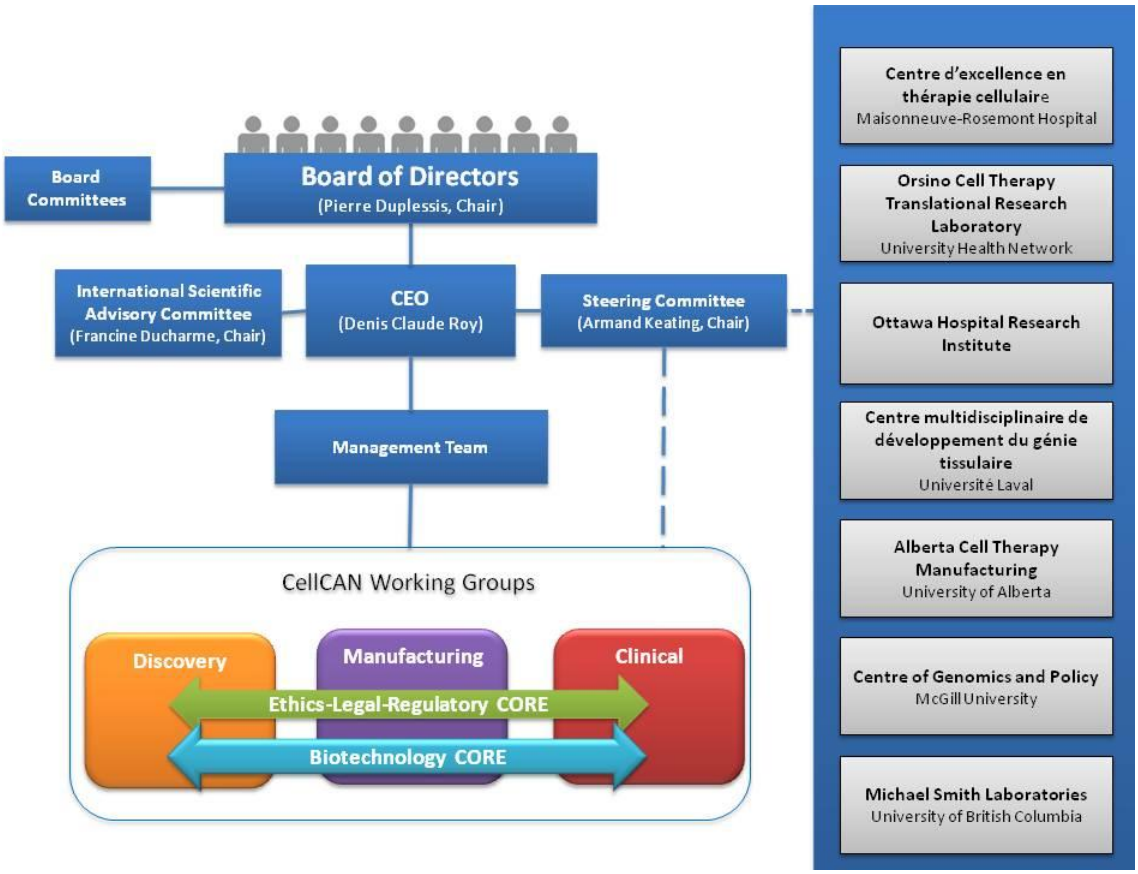
The governing body of CellCAN is the Board of Directors. In accordance with NCE's rules, members of the Board are prominent individuals in the field of governance, finance, legal and

[1] The others pillars being *pharmaceuticals*, *biologics* and *medical devices*.
Mason C., Brindley, D.A., Culme-Seymour E.J. and Davie N.L. (2011). **Cell therapy industry: billion dollar global business with unlimited potential. Regenerative Medicine**, May 2011, Vol. 6(3), 265-272.

knowledge mobilization. Directors are appointed by the Members in a general assembly, a procedure common to non-profit organizations legally constituted.

The Board is supported by a Chief Executive Officer (CEO) who is responsible for management and insure efficiency and rigor in activities and fund management, in the context of a multicenter and multidisciplinary organization.

To ensure the representation of participating institutions, a Steering Committee was set up with the aim to advise the CEO on the development and execution of strategies regarding CellCAN's activities. This committee comprises representatives from each of the seven Founding Network Affiliates.



Graphic A – CellCAN's Organizational Structure

2 STRATEGIC ANALYSIS

2.1 Key concepts

Before we begin with the strategic analysis, here are some definitions of key concepts.

Stem cells are undifferentiated cells that can divide many, many times, and still be capable of developing into specialized cells within the human body, such as blood, nerve, heart muscle, pancreatic islet cells and bone.

Cell-based 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 refers to the innovative methods to replace or regenerate damaged human cells, tissues, or organs to restore or establish normal function. It is supported by a number of diverse technologies including tissue engineering, developmental and stem cell biology, gene therapy, cellular therapeutics, biomaterials (scaffolds and matrices), nanoscience, bioengineering and chemical biology. Cell (and stem cell) therapy is therefore just one element - but a major component - of regenerative medicine.

2.2 General Environmental Analysis

2.2.1 Canadian cell-based therapy Industry

Canada has a relatively small but thriving group of innovative cell-based industrial companies. To date, more than 20 companies have been identified as active participants in the sector based on a search in the BIOTECanada database. A further seven companies operate in the broader regenerative medicine space. The industry predominantly consists of SMEs with sustainability as a major concern. That being said, there are some highly successful Canadian stem cell-based technology companies.

One of Canada's most successful life sciences companies is STEMCELL Technologies, which employs more than 500 people and delivers over 1,500 products to almost 80 countries. Its focus is the provision of standardized reagents and tools for all areas of stem cell research, including cell isolation, culture substrates and matrices to promote cell attachment and growth as well as serum substitutes to supplement and provide nutrients for cell survival, growth and division.

2.2.2 Canada's leadership and political context

Canada is well positioned to lead the next wave of development as the global cell therapy and regenerative medicine industry undergoes exponential growth over the next decade. Following the way paved by Drs. James Till and Ernest McCulloch in the early 1960s, Canadian scientists continue to build on this legacy as world leaders in stem cell research.

Our historic strength in stem cell research, welcoming business environment for R&D, highly educated and multicultural workforce, and investment in this area over the last decade has positioned the country as a formidable player, especially in research and early stage development. This was further supported by significant investment. Indeed, between 2006 and 2011, Canada invested more than $227.5 million mostly in stem cell and regenerative medicine infrastructure for research. The majority came from the Canadian Institutes of Health Research (CIHR) and Canada Foundation for Innovation (CFI), who provided 36% and 23% of funds respectively.

For several years now, the federal and provincial investments in research have been systematically reduced, partially due to the economic context. This has created an unfavourable context for research and innovation in Canada. Furthermore, cell-based therapies in Canada still face some conservative views towards stem cells research, a legacy of the fear of human cloning and of misuse of embryonic cells.

2.2.3 Stem Cell Hype

The ground breaking scientific discoveries and innovations around cell-based therapies lead to great enthusiasm about the benefits that may flow. One danger, however, is that enthusiasm may lead to hype, or exaggerated publicity and extravagant claims. Hype can lead to unrealistic expectations of both the benefits and the speed at which they will be achieved.

Stem cell researchers have walked a fine line between enthusiastically describing the long-term potential of stem cell therapies, which helps get support for their work and in cautioning that science and research take time and sustained support. Part of the tension for researchers, lies in the need to work slowly and carefully, and yet also to attract industry funding based on the potential commercial and clinical applications from their work.

Canada has adopted the self-regulatory model in the form of provincial medical colleges (Colleges of Physicians and Surgeons) constituted and run by members of the medical profession.

A SCN-funded review by researchers at the University of Alberta of internet sites offering stem cell therapies concluded that in general “indications for therapy are indeterminate or over-broad, benefits are overstated, risks are understated, and certainty of knowledge in the field is inflated.” In order for a patient to give informed consent to any stem cell therapy or research trials, the risks need to be discussed and understood. The International Society for Stem Cell Research (ISSCR), a leading professional group, has issued a guide for patients and their families advising them to approach stem-cell therapy with extreme caution.

Some countries such as China, Mexico and Costa Rica have less oversight of both stem cell research and therapies and are becoming havens for stem cell tourism. Stem cell tourism involves travelling, often very far away, to pay for stem cell therapies that are not available in

one's own country. Examples of stem cell tourism abound in the news and often involve methods that are not scientifically approved.

2.2.4 The economic benefits of Cell-based Therapy

Over the past few decades, conventional pharmacological treatments have allowed clinicians to cure or control numerous acute and chronic diseases. Despite this success, there are many diseases for which there is no cure. Furthermore, changing demographics will very soon lead to a significant increase in people over the age of 65, which means an increase in age-associated human disorders (such as heart diseases, cancer, stroke, diabetes, bone diseases and others) as well. Significant cost is associated with treating these diseases. For instance, in the US, the direct costs associated with heart diseases is $273 billion (2010) and is projected to increase to $818 billion in 2030 (representing 17% of health care cost). Treating rare or common disorders with regenerative cell based therapy has tremendous potential.

In 2011, the stem cell therapies market was valued at $2.7 billion, consisting almost entirely of revenues from the well-established bone marrow transplant segment. Furthermore, stem cell biobanking and ancillary products were estimated to be worth $2.6 billion. Stem Cell therapies are advancing across a broad and diverse front, and the total market is expected to expand to $8.8 billion by 2016. This expected market growth equates to a compound annual growth rate of 12.6% for the period between 2011 and 2025.

2.2.5 Health Care Systems

Large-scale corporate investments in the field have been slow to materialize, largely because of the complexity of cell and tissue - based therapies compared with small molecules and even other biologics; a cautious and evolving regulatory pathway; and the lack of proven business and investment models.

Health care systems have to adopt methods for health technology assessment to ensure that novel therapies are more cost effective than the ones they replace within very limited healthcare budgets. Given the personalized nature of many intended applications for regenerative medicine, as well as manufacturing costs, which may exceed even those for biopharmaceuticals, cell – and tissue - based therapies will likely need to be close to curative to be cost effective compared with current therapies and thus be considered for adoption by healthcare systems. Alternatively, they must target diseases for which there are limited or no treatment options, to which standard value criteria are less stringently applied. Indeed, cutting edge health innovations such as cell-based therapies are deemed expensive for the health care system, even with forecasted short to mid-term savings.

2.2.6 Significant investments in some prominent countries

Recognizing the need to invest in clinical research and commercialization resources, other jurisdictions have begun to build the assets required to secure their place in the global cell therapy and regenerative medicine sector:

- Japan has recently allocated significant funding (as part of a broader $3-billion stem cell strategy) for stem cell research based on its potential for commercial, industrial and clinical applications.
- In Europe, the UK's Cell Therapy Catapult is helping businesses bring innovative ideas to commercialization through targeted efforts to address challenges in each of the business model, manufacturing and supply chain, and clinical and regulatory frameworks.
- In the US, the California Institute for Regenerative Medicine (CIRM) plans to invest $70 million over five years in an extensive network of Alpha Clinics as part of California's stem cell strategy. These specialized centres take advantage of the expertise and existing facilities in academic medical centres to conduct early phase clinical trials of stem cell therapies.
- Major US universities, such as Harvard, Stanford and Columbia, have also launched multidisciplinary stem cell initiatives and/or institutes with a focus on translating discovery to clinical practice.
- The National Health and Medical Research Council in Australia provide grants for health technologies that show potential for commercialization.

2.2.7 Canadian Stem Cell Strategy

A proposal for a Canadian Stem Cell Strategy was recently presented to the federal government. This Strategy stems from a coalition led by the Canadian Stem Cell Foundation, SCN and CCRM. CellCAN was involved in the preparation of the strategy, Dr Denis Claude Roy leading a clinical research working group. The Strategy is calling on Ottawa to commit half a billion dollars over the next 10 years to boost stem cell research and development in Canada, aiming at making stem cell science a national priority, to enable Canada to fully reap the socio-economic benefits of cell-based therapies.

CellCAN could be pivotal putting this Strategy in action, as the go-to resource throughout Canada for cell and tissue manufacturing. This would help basic research, preclinical research as well as clinical trials.

2.3 CellCAN specific environmental analysis

2.3.1 Ethic and regulatory context

Stem cell research in Canada is governed by the principles of free and informed consent, upholding human dignity, concern for the health and welfare of all human beings, the non-

commercialization of gametes and embryos, respect for privacy and confidentiality and for any laws that limit the use and creation of embryos. Several studies have noted the existence of a direct relationship between regulation and innovation in the clinical translation and commercialization of scientific discoveries and how regulatory frameworks could enable or hinder innovation.

The Canadian regulatory context is considered as being favourable for cell-based therapy. Indeed, Canada is the first Country to have adopted Prochymal in 2012 thanks to accelerated licensing supported by a Notice of Compliance with Conditions (NOC/c) policy. A new Orphan Drug regulatory framework is also under development. These regulations allow the application of a risk-benefit approach to all drug regulatory processes and are sufficiently flexible to enable the development of novel products. There are also accelerated pathways that can be applied to cell therapy and gene therapy development.

2.3.2 Clinical research

Clinical research drives innovation and is central to the development of world leading health care practice and high standards of patient care. It provides a multitude of benefits, including:

- advances in clinical therapy expertise;
- economic prosperity through industrial investment (direct and indirect job and company creation);
- access to novel therapies for patients who have limited options available to them.

As mentioned by the experts we consulted, Canada is a highly desirable place for clinical research and has created a sophisticated industry over the last few decades. The country offers highly qualified clinicians supported by experienced clinical research staff, state-of-the-art research facilities, a diverse patient population, as well as generous R&D tax credits that make it a relatively cost efficient destination.

2.3.3 Research infrastructures

Canada is home to many active research centres affiliated with universities and/or hospitals with expertise that range from basic research, assay development, toxicology, project management, manufacturing, quality control, biostatistics, ethics, regulation, clinical research and design of clinical trials.

Cell therapies require extensive cell manufacturing infrastructure to manipulate, process and store cells in preparation for clinical application. Such production requires elaborate bio manufacturing processes and equipment that are unique to this field and which are expected to operate under cGMP standards.

2.3.4 Cell and tissue manufacturing facilities and their primary areas of expertise

In recent years, close to $100 million of federal, provincial and philanthropic funds have been invested in building new and unique specialized GMP (Good Manufacturing Practice) cell manufacturing facilities across the country, providing Canada with close to 30 new Health Canada and FDA compliant clean rooms.

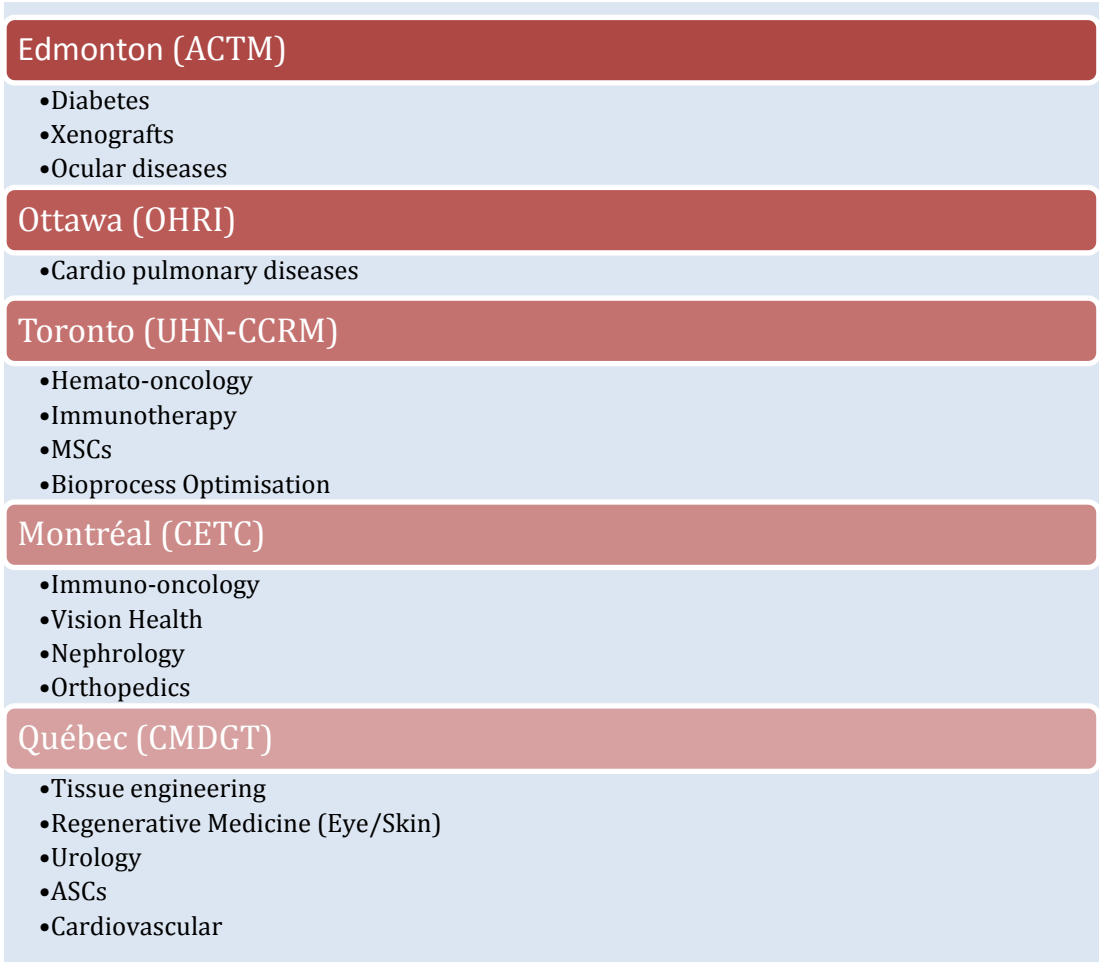
All of these centers are performing world-class science and are producing the most innovative cell and tissue products, as confirmed by the fact that all of these facilities have received major CFI funding.

- These cell therapy centers have the capacity to conduct 50-75 new Phase I/II cell-based clinical trials over the next five years.
- They represent a combined capacity of over 4500 m2 with the ability to isolate, grow and manipulate cells in a tightly controlled (temperature, humidity, pressure, particles, etc.) and highly regulated and monitored environment ensuring their safe and traceable administration to patients across Canada.
- The entire clinical manufacturing facilities have controlled access requirements and are isolated from other operations in order to preserve the integrity of products and reduce possibilities of cross-contamination.
- More than 50 highly qualified personnel support these centers.

The breadth of know-how encompassed by the cell manufacturing facilities within CellCAN represents an outstanding resource for the rapid and efficient development of cell-based therapies in Canada. According to several stakeholders, CellCAN represents a network with unprecedented linking not only of cell manufacturing facilities, but also of the scientific community of stem cell researchers and clinician-scientists, along with patients and general public.

Two transversal cores support the work of the Cell & Tissue Manufacturing Facilities:

- The Centre for Genomics and Policy at McGill University, devoted to developing best practices, as well as regulatory and ethical-policy guidance for regenerative medicine and cell therapies, amongst other fields
- The UBC- Biotechnology Core which aims at improving the design and operation of stem cell and tissue manufacturing by ultimately establish robust and non-invasive culture process controls through analysis, prediction and validation of process performance for clinical grade production.



Graphic B – Primary areas of expertise of Cell and tissue manufacturing facilities

2.3.5 Commercialization of regenerative medicine

Canada excels at scientific research. Our consultation with the industry stakeholders confirms that there is a growing opportunity to transform our rich knowledge base into commercial products, allowing companies to deliver new therapies to patients and create wealth for all Canadians.

Canada ranks third in the world in terms of academic-based research investment relative to gross domestic product (GDP) and quality of research, but it only ranks 14th overall in the World Economic Forum's Global Competitiveness Index in 2012/13 (down from 12th in 2011/12). Gaps in the commercialization pipeline and funding are key challenges explaining this.

2.4 Overview of key stakeholders

The uniqueness of this strategic plan is that goals and objectives are directed towards key stakeholders. This is inherent to our mission and the very nature of the organization.

This section of the strategic analysis deals precisely with the identification of key stakeholders.

2.4.1 CellCAN's Network Affiliates

The CellCAN's Network Affiliates are the five cell and tissue manufacturing facilities, and the two transversal cores that support the work of the cell and tissue manufacturing facilities. They were the initial founders who asked to form a KM network.

2.4.2 Scientific community

The scientific community are at one end of the stakeholder spectrum. They initiate research on many diseases and they need to translate those researches onto clinical trials. Communications between the scientific community and the cell and tissue manufacturing facilities is not always easily accessible.

2.4.3 Organizations evolving in the cell-based therapy network in Canada

A number of national and regional organizations and networks – like CellCAN – have been established and have made significant contributions to the stem cell and regenerative medicine sector. These organizations have moved the stem cell agenda forward by fostering national and international collaborations and partnerships, supporting the translation and commercialization of stem cell discoveries, and promoting public awareness (see appendix 1).

Although several of these organizations gather some key players, our consultation with stakeholders underlines the fact that there is not, to date, a single "Go to" resource for cell-based therapy knowledge producers and end users communities to efficiently interact.

Noteworthy, the Stem Cell Network is now phasing out as the funding of the program is terminating in 2017. This leaves a space that needs to be occupied, as well as an opportunity for CellCAN to build on the SCN legacy.

2.4.4 Patients and patients advocacy groups

As the field of cell-based therapy comes to maturity, patients and health care providers have gathered to create advocacy groups, united by a disease or disease family. These groups represent a unique platform for dialogue with patients, and their families, aiming towards better education and knowledge access for novel treatment options.

2.4.5 Healthcare professionals

Healthcare professionals are the ones who'll likely prescribe the novel cell therapies in the years to come, so they need to be aware and informed about available and upcoming therapies. They also are the first line of response for patients. And as such they play a key role in disseminating the information. Finally, they have a key role to put pressure on policy makers and regulatory bodies to make cell therapies accessible.

2.4.6 Lay public

Everyone is concerned about health issues, either personally or because someone close is living with a disease. The level of knowledge in the lay public is different from one to another and a lot of myths about cell therapies and the actual state of science are circulating. The lay public needs to be mobilized and even more engaged, not only to put pressure on the policy makers, but also to be careful and aware of real treatments available.

2.4.7 Policy makers and regulatory bodies

In order for innovative cell therapies to be readily available to patients in Canada, policy makers and regulatory bodies need to put in place the proper framework to evaluate them. This will allow their integration in our health care system, both provincial and federal. To this end, a close dialogue is necessary.

2.4.8 Potential investors and partners

Investment is key to advance research and its translation into novel cell therapies. Showcasing the potential socio-economic benefits of cell-based therapies is essential to galvanise those critical investments.

2.5 SWOT Analysis

In this section, major and most meaningful findings of the strategic analysis are captured into a SWOT table. It provides a clear vision of our organization's **S**trengths and **W**eaknesses and summarizes the **O**pportunities and **T**hreats in the environment. The SWOT table thus concludes our strategic analysis.

Organization's Strengths	Organization's Weaknesses
• Expertise • Governance • Accountability framework • Performance measures and risks and mitigation strategies • Well positioned to address several of the challenges in the cell-based	• Lack of a management plan to bring the institutions together in a consortium model. • Lack of access policy to the services provided by the Cell and Tissue Manufacturing facilities. • No framework agreement between the

therapy field, including the scale-up of production and development of standardized operating procedures.	institutions to define parameters and modus operandi for fund distribution and overall collaboration • No common ethics and regulatory guidance

Environment's Opportunities	Environment's Threats
• Become the central hub for cell-based therapy in Canada • Channel the excitement from the scientific and medical community, the patient advocacy groups and the lay public towards cell-based therapy • Synergize the activities, resources and knowhow of cell-based therapy in Canada • Support Canada's leadership in major international cell-based therapy initiatives • Facilitator of socio-economic benefits of cell-based therapy research • Contribute to Canada's world leader position in the cell-based therapy field • Facilitator of the sustainability of past investment in cell-based therapy infrastructures in Canada • Catalyst of translation of innovation to the clinic • Building on the SCN legacy after the end of the program in 2017 • NCE competition in 2017 • Obtain federal and provincial funding to support cell-based therapy • Incorporation of new cell and tissue manufacturing facilities within the CellCAN network • Develop strong links with key networks in the field of cell-based therapy such as BioCanRx and CCRM	• Difficult economic context • Lack of active involvement of stakeholders /Possibility for CellCAN and other organizations to have overlaying expertise and initiatives in certain aspects of their activity • Competition between cell & tissue manufacturing facilities • The Valley of death of funding in particular for clinical trials • Lack of funding for research, operations, clinical trials for cell and tissue manufacturing centers • CellCAN branding/leadership • Overlapping role/mission/objectives of some of the organizations in the cell-based therapy arena in Canada (e.g. CCRM, CSCF) • Perception of high cost of cell-based therapy and scepticism about clinical utility/Cost of health care system with an aging population • Cell-based therapy hype and stem cell tourism • Unresolved regulatory and ethical issues • Increased competition from other countries

3 MISSION AND STRATEGIC GOALS

This strategic analysis demonstrates that CellCAN's mission is still relevant and doesn't need to be updated. This being said, we find that to fully fulfill that mission, we need to revisit our strategic goals.

3.1 New strategic goals

At the time of its inception, CellCAN set **6 strategic goals**, which are stated in the NCE Network Agreement:

1. Standardize regenerative medicine and cell therapy by responsible sharing data generated at various Canadian facilities;
2. Accelerate the implementation of novel regenerative medicine and cell therapy applications;
3. Proactively engage with stakeholders involved in regenerative medicine and cell therapy;
4. Promote the implementation of innovative cell therapies by disseminating knowledge to clinical centers, industrial partners as well as to patients and the public at large;
5. Reduce operational costs and enable more rapid technological advances; and
6. Enable Canadian researchers and clinicians to collaborate with the international scientific community.

However, these goals did not allow us to properly define a knowledge mobilization strategy and measurement framework, as they overlapped to a great degree and were not conducive to establishing clear outcomes and metrics.

By engaging in a strategic planning effort, CellCAN has set **3 new strategic goals**:

1. **Provide cell manufacturing facilities with the most up to date best practices in order that they can produce and deliver high-quality cell-based clinical trials and therapies in Canada.**
2. **Engage stakeholders in cell-based clinical trials and therapies.**
3. **Become the enabler of cell-based clinical trials and therapies in Canada.**

4 STRATEGIC OBJECTIVES AND EXPECTED OUTCOMES

This section lists for each strategic goal, the strategic objectives, metrics, targets and expected outcomes. Targets are specified for the majority of metrics. Otherwise, targets indicate in what direction the metric in question must evolve. These targets will be determined in the coming years depending on the availability of information.

4.1 Goal 1: Provide cell manufacturing facilities with the most up to date best practices in order that they can produce and deliver high-quality cell-based clinical trials and therapies in Canada

Target stakeholders:

- CellCAN's network affiliates (Canadian cell manufacturing facilities and cores)
- Policy makers and regulatory bodies

Strategic objectives	Metrics	Targets	Expected outcomes by 2020
1.1 Enable the establishment of a collaborative culture between CellCAN network affiliates	# of collaborative projects # of shared resources and usage of the data warehouse by facilities # of similar projects	Increase All network affiliates share data, know-how and participate in the discussion groups Decrease	Most project developments benefit more than one cell manufacturing facility Increased synergy between facilities
1.2 Harmonize procedures between the cell manufacturing facilities	% of harmonized procedures identified by the Steering Committee % of harmonized procedures used by centers	75% 30%	Better results in CellCAN network affiliates yearly performance audit Global savings in the development and update of procedures
1.3 Facilitate communications and partnerships within the cell therapy ecosystem including CellCAN network affiliates	# of new partnerships # of networking opportunities # of new facilities joining the network	Increase Increase Increase	Availability and use of a unique platform for knowledge mobilization in cell therapy
1.4 Improve knowledge of CellCAN network affiliates	Training opportunities initiated by CellCAN and identified by the Steering Committee Updates in CellCAN data warehouse Appreciation of CellCAN training activities by participants	Opportunités qu'ils n'auraient pas autrement Continuously 85% satisfied	Savings in training expenses for all network affiliates Highly specialized know how in cell therapy widely available in Canada Better quality and quality control across Canada

1.5 Influence policy makers and regulatory bodies	CellCAN is involved in various advocacy groups and engaged with regulatory authorities	1 CellCAN representative in the working group with Health Canada	Evolution and better adaptation of regulation for the development of cell therapies in Canada

4.2 Goal 2: Engage stakeholders in cell-based clinical trials and therapies

Target stakeholders:

- Patients
- Scientific community
- Organizations evolving in the cell-based therapy network
- Lay public
- Healthcare professionals

Strategic objectives	Metrics	Targets	Expected outcomes by 2020
2.1 Improve stakeholders knowledge	Survey every 2 years in particular before and after KM events # of questions received via the KM Portal Help Desk # of answers to the questions received from the KM Portal Help Desk	Improvement Increase Increase	The various target audience have a better understanding of cell-based therapy

4.3 Goal 3: Be the enabler of cell-based clinical trials and therapies in Canada

Target stakeholders:

- Scientific community
- Organizations evolving in the cell-based therapy network
- Lay public
- Healthcare professionals
- Policy makers
- Potential investors and partners

Strategic objectives	Metrics	Targets	Expected outcomes by 2020
3.1 Raise awareness about the socio-economic benefits of cell-based therapy	# of major investments in cell-based therapy from private or public sectors, locally, provincially and nationally	Increase	Cell therapy is a key economic driver in the health sector in Canada
3.2 Become the go-to information hub for cell-based clinical trials and therapies in Canada	KM Web portal and datawarehouse consultations statistics # of formal requests for sharing information from partners Invitation as a spokesperson of Canada in an international event	Annual increase Increase At least 1 per year	Recognition by network affiliates, partners, collaborators, and by the media and the lay public as a leader in information on cell therapy in Canada

5 PERFORMANCE ASSESSMENT

CellCAN is a not-for-profit corporation funded by the federal government, augmented by in-kind contributions and revenues generated by the provision of services. The underlying principles of the Governance and Management of CellCAN have been crafted to reflect principles of good governance including:

- Performance Evaluation: processes and structures in place produce results that meet objectives of the organization while making the best use of resources

- Accountability: Decision-makers are accountable to the public as well as to stakeholders. Transparency is built on the free flow of information – processes, structures and information are directly accessible to those concerned with them, and enough information is provided to understand and monitor them.

The following main bodies provide performance monitoring within CellCAN.

Board of Directors

The principal governing body is the Board of Directors. The primary role of the Board of Directors (the "Board") of CellCAN is the stewardship of the organization. The Board's fundamental objective is the protection and enhancement of the value of the organization's assets. The Board oversees the conduct of the organization's business and supervises management, which is responsible for the day-to-day conduct of the business. In supervising the

conduct of the business, the Board, through the Chief Executive Officer, sets the standards of conduct for the organization.

The management team has implemented a project management approach to support its operations. This is critical considering the few resources of the organization. This approach will allow the production of a scorecard that the Board will use to follow the progress of annual objectives and overall strategic goals. Metrics linked to each objective allow for a quantitative and qualitative progress evaluation and for readiness in identifying risks and propose mitigation strategies to address.

Steering Committee

The Steering Committee advises the CEO on the elaboration and execution of CellCAN activities. It regroups CellCAN's founding affiliates who are representative of the main cell therapy facilities in Canada. The Steering committee is a critical support to the CEO and management team in providing expert advice on the KM needs of the scientific community. It is also the source of expertise for the material produced by CellCAN to engage with various stakeholders.

International Scientific Advisory Committee (ISAC)

The ISAC advises on emerging science and technology trends, challenges and opportunities in national and international contexts of cellular therapies. The ISAC will advise on scientific and business partnerships and linkages that could help support CellCAN's mission. Furthermore, the ISAC will be performing a mid-term performance review of CellCAN.

NCE Steering Committee and NCE Monitoring Committee

The NCE programs are overseen by a tri-agency NCE Steering Committee made up of the Deputy Minister of Industry and Health (or delegate), the presidents of the three granting agencies, the Natural Sciences and Engineering Research Council (NSERC), the Canadian Institutes of Health Research (CIHR), and the Social Sciences and Humanities Research Council (SSHRC), and the president of the Canadian Foundation for Innovation (CFI) (as an observer). In accordance with the Funding Agreement, the progress of CellCAN is reviewed on an annual basis by the NCE Monitoring Committee who makes recommendations to the NCE Steering Committee with respect to whether the network: (a) should continue its activities, (b) should phase-out its NCE funding or (c) should undergo in-depth review by an Expert Panel. The role of the NCE Monitoring Committee is as much acknowledging progress and highlighting excellence as well as identifying areas for improvement that will ensure the continued progress of the network.

Other means

In addition, CellCAN will use various KM tools to monitor within and outside the network the needs, satisfaction and impact of CellCAN's activities such as surveys, focus groups workshops etc.

6 CONCLUSION

All stakeholders consulted agree on the fact that we are at the dawn of a new era in health care, the enormous potential of stem cell research and regenerative medicine is about to be realized. In the past five to 10 years, we have witnessed the development of several novel cell therapies. Although therapies for complex diseases such Alzheimer's remain many years from the clinic, for other diseases like blood cancer, novel cell therapies are now becoming standard of care, while numerous others like glaucoma and cardiopulmonary diseases will be added as they are now entering clinical trials.

Cell-based therapy is transforming the practice of medicine from the traditional model of continual interventions to address symptoms to curative treatments. The unique nature of cell-based therapy thus offers a new health paradigm with the potential to definitively address root causes of illness and disease.

This strategic analysis demonstrates that:

- CellCAN is well positioned to facilitate the reaping of the fruits of Canada's scientific leadership in cell-based therapy and of the major investments made in the recent years.
- The multiple players in the cell-based therapy arena in Canada together with its important potential for economic, political and social impacts create the need for a unique "go to" resource. CellCAN should aim to become this natural enabler and hub for cell-based therapy in Canada
- As a network gathering scientists, clinicians, hospitals and cell and tissue manufacturing facilities, CellCAN is uniquely positioned to help galvanise cell-based therapy clinical trials in Canada. This is very timely, as cell-based therapy is reaching a level of maturity where numerous innovative therapies are quickly advancing the pipeline of clinical development.

The unoccupied space left by the end of the Stem Cell Network in 2017 leaves an important opportunity for CellCAN. It almost seems natural for CellCAN to occupy that vacant space and to build on the SCN legacy, thus enabling CellCAN to fully participate in the implementation of the Canadian Stem Cell Strategy.

We are deeply committed to achieving the three goals in this plan and convinced that in so doing CellCAN will become the key enabler of cell-based therapy in Canada.

APPENDIX 1: ENGAGED STAKEHOLDERS

Name	Organization
Denis Claude Roy	Center of Excellence in Cell Therapy (CETC)
Armand Keating	Orsino Cell Therapy Translational Research Laboratory
Sowmya Viswanathan	Orsino Cell Therapy Translational Research Laboratory
Duncan Stewart	Ottawa Hospital Research Institute
David Courtman	Ottawa Hospital Research Institute
Lucie Germain	Centre Multidisciplinaire de Développement du Génie Tissulaire
François Auger	Centre Multidisciplinaire de Développement du Génie Tissulaire
Friederike Pfau	Centre Multidisciplinaire de Développement du Génie Tissulaire
Greg Korbutt	Alberta Cell Therapy Manufacturing in Edmonton
Gayle Piat	Alberta Cell Therapy Manufacturing in Edmonton
Bartha M. Knoppers	Centre of Genomics and Policy
Rosario Isasi	Centre of Genomics and Policy
James Piret	Michael Smith Laboratories
Michael Rudnicki	Stem Cell Network
Phil Welford	Stem Cell Network
Alan Bernstein	Canadian Institute for Advanced Research
Jacques P Tremblay	CRCHUQ-U. Laval
John R. Gordon	University of Saskatchewan
Michael May	CCRM
Stacey Johnson	CCRM
Nick Trimmins	CCRM
Janet Rossant	Hospital for Sick Children in Toronto
Peter Zandstra	CCRM-U of Toronto
Donna Wall	Manitoba Institute Child Health
Kirk Schultz	BC Children's Hospital and the Child and Family Research Institute
Michael Kallos	University of Calgary
Molly Shoichet	University of Toronto
Lori West	CNTRP
David Hartell	CNTRP
Stephanie Maier	CNTRP

Name	Organization
Marc Lussier	CETC
Martin Giroux	CETC
Jean-Sébastien Delisle	Hôpital Maisonneuve-Rosemont
Joachim Madrenas	McGill University
Jonathan Bramson	McMaster University
Keith Humphries	UBC
Tania Bubela	University of Alberta
Megan Levings	The University of B.C.| Child and Family Research Institute
Francine Gendron	Myeloma Canada
Denis Bilodeau	Myeloma Canada
Lucy Di Carlo	Leukemia Lymphoma Society
Lorna Warwick	Leukemia Lymphoma Society
Christine Janus	Skin Patient Alliance
James Price	Stem Cell Foundation
Joe Sornberger	Stem Cell Foundation
Lori Lyons	Fighting Blindness Foundation
Judy Iles	UBC-NeuroDevNet
Anneleise Poetz	York University-NeuroDevNet
Brendan McIntyre	Mylteni
Adil Shivji	Stem Cell Technologies
Aldo Del Col	Patient
Michel Lamoureux	Patient
Tim Caufield	University of Alberta
Marie-Josée Hébert	University of Montreal
Guy Sauvageau	IRIC
Claude Perrault	University of Montreal
Francine Ducharme	Ste-Justine Hospital
Connie Eaves	UBC
Sam Donaldson	Ontario Stem Cell Initiative
Michèle Savoie	Montréal In Vivo
Melanie Barwick	Canadian Knowledge Transfer and Exchange Community of Practice

Name	Organization
Peter Levesque	Canadian Knowledge Transfer and Exchange Community of Practice
Jerome Ritz	Harvard University
Adrian Gee	Baylor College of Medicine
Chris Mason	University College London
Philippe Hénon	CellProthera

APPENDIX 2: ORGANIZATIONS AND NETWORKS INVOLVED IN CELL-BASED THERAPY

Stem Cell Network of Centres of Excellence (SCN), founded in 2001, is pivotal in creating a collaborative, multidisciplinary culture for stem cell research in Canada. It has supported seven Phase I and II clinical trials in stem cell and regenerative medicine research since 2008 using Government of Canada funding (approximately $5 million annually) and an equivalent amount from charities, provincial governments and industry. It is a member of the International Consortium of Stem Cell Networks that aims to unify international efforts to accelerate opportunities to make stem cell therapy a reality for patients and the public.

The Canadian Stem Cell Foundation is a privately funded, independent, registered charitable organization. As the national champion and advocate for stem cell research, the Canadian Stem Cell Foundation works to align scientists, clinicians, industry, business and community leaders and the public to accelerate the development of new treatments and cures for devastating diseases. Long term, the Foundation's success will not only save lives and reduce suffering, but it will also improve health care and create economic benefits for Canada. The Foundation is committed to realizing the stem cell promise for Canada and the rest of the world.

The Centre for Commercialization of Regenerative Medicine (CCRM) is a Centre of Excellence for Commercialization of Research established in 2012. Its goal is to translate promising discoveries into commercially viable regenerative medicine products to accelerate the growth of the industry. It has formed an industry consortium of Canadian and international companies and has established three technical platforms to support stem cell science translation. CCRM also provides business support to its industry partners, conducts technology evaluation, helps bundle intellectual property into marketable products and engages venture capital sources.

Ontario Institute for Regenerative Medecine (OIRM) builds on the strengths of OSCI as a network of scientific leaders and CCRM as a network of industry and business leaders. Following the official announcement of OIRM, OSCI began to wind down, allowing OIRM to integrate and align existing resources and infrastructure for the overall benefit of Ontarians.

Ontario Stem Cell Initiative (OSCI) is a virtual network that encompasses over 130 stem cell and regenerative medicine research programs across nine centres in Ontario. OSCI represents a direct portal to stem cell activities all over the province, informing and engaging key stakeholder groups which include research partners, industry, government, funding agencies and the international

scientific community.

ThéCell is a research network aimed at promoting translational research in cell and tissue therapy in the Province of Quebec.

The **Canadian Institutes of Health Research** is the Canada's federal funding agency for health research with a mission to create new scientific knowledge and enable its translation into improved health, more effective health services and products, and a strengthened Canadian health care system. It consists of 13 institutes and provides leadership and support to health researchers and trainees across the country.

The **Centre for Drug Research and Development** is a national drug development and commercialization centre aimed to de-risk discoveries stemming from publicly funded research to create viable investment opportunities for the private sector. It is a fully integrated centre with the expertise and infrastructure to source, evaluate, develop and commercialize small molecules and biologic innovative technologies.

Canadian Blood Services' National Public Cord Blood Bank is a division which collects, manufactures and stores donated cord blood-derived stem cells for the treatment of any Canadian or international patient in need of a transplant. The Bank also provides biomedical researchers with cord blood-derived stem cells unsuitable for transplant for research purposes.

The **Canadian National Transplant Research Program** is a world's first national research network that brings together solid organ transplant, hematopoietic cell transplant and donation, and critical care research communities from across the country.

The **Institute for Research in Immunology and Cancer – Commercialization of Research (IRICoR)** is a Centre of Excellence for Commercialization and Research (CECR) that supports the development of novel small molecules for stem cell expansion and new stem cell therapies.

Health Charities Coalition of Canada (HCCC) is a membership-based organization that is dedicated to advocating for sound public policy on health issues and promoting high-quality health research. Apart from co-funding with the government on important health research initiatives, HCCC seeks to ensure that policy makers look to the Coalition for timely advice on major health issues affecting Canadians

Ontario Bioscience Innovation Organization (OBIO) is a membership-based organization that is involved in developing an integrated health innovation economy for Ontario. Through advocacy, strategic leadership and collaborative partnerships, OBIO strives to become a global leader in the provision of health technology products and services.