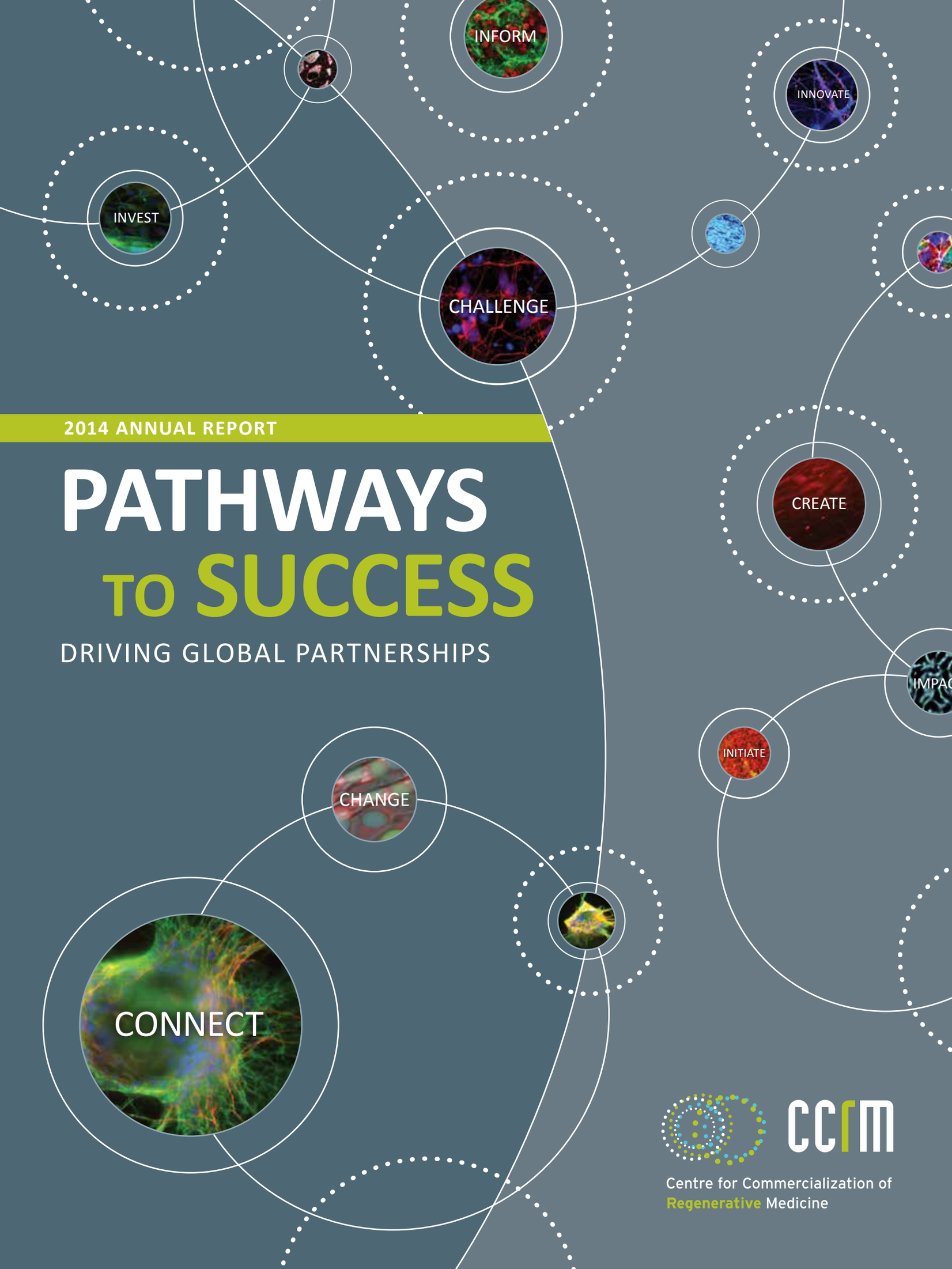


2014 ANNUAL REPORT

PATHWAYS TO SUCCESS

DRIVING GLOBAL PARTNERSHIPS

ccrm

Centre for Commercialization of Regenerative Medicine

CONTENTS

Centre for Commercialization of Regenerative Medicine (CCRM) is a Canadian, not-for-profit organization supporting the development of foundational technologies that accelerate the commercialization of stem cell- and biomaterials-based products and therapies. CCRM is supported by the Centres of Excellence for Commercialization and Research (CECR) program.

Regenerative medicine, which aims to harness the power of stem cells, biomaterials and molecules to repair, regenerate or replace diseased cells, tissues and organs, has the promise to treat, manage and perhaps cure some of the most devastating and costly diseases in the world today.

Many new and potentially life-changing regenerative medicine-based treatments never reach patients because they are not successfully moved from the laboratory to the clinic. In order to fulfill regenerative medicine's promise to treat the many diseases affecting our population, a world-renowned group of stem cell scientists and bioengineers has come together to form CCRM.

We ENABLE
product development through unique translational platforms that address the key bottlenecks in regenerative medicine commercialization.

PAVING THE WAY FOR REGENERATIVE MEDICINE

CENTRE FOR COMMERCIALIZATION OF REGENERATIVE MEDICINE (CCRM) is a network that bridges the regenerative medicine commercialization gap by leveraging funding and infrastructure, and mobilizing business and scientific expertise to translate technologies into commercial products and therapies. CCRM's mission is to create and sustain a global nexus for company creation, technology and cell therapy development, and clinical trials in regenerative medicine.

We INTEGRATE
Canada's strength in stem cell and bioengineering sciences with dynamic business leadership.

We ENGAGE
through a consortium of leading companies and international partners that provide market pull and act as receptors.

EXECUTIVE MESSAGE:
IT'S BEEN A GREAT RIDE SO FAR

It seems like just yesterday that the Centre for Commercialization of Regenerative Medicine (CCRM) was awarded $15 million by the federal government to operate as a Centre of Excellence for Commercialization and Research (CECR).

In reality, we have accomplished a great deal since we formally launched in June 2011 and this annual report captures significant activities that took place from January to December 2014. (Please visit the Media Centre page at www.ccrm.ca for a report that covers our launch to the end of 2013.)

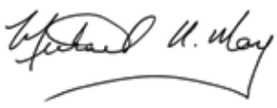
We are excited to be applying to the Networks of Centres of Excellence for a 2nd mandate while we have so many exciting projects in development.

Some of our future plans are included in the following pages, or at least hinted at, and others will be shared when we've attained significant milestones. We are very pleased that, in partnership with the Ontario Stem Cell Initiative and with funding from the Province of Ontario, we launched the Ontario Institute for Regenerative Medicine in November 2014. This new institute will take a tremendous leadership role in funding and facilitating stem cell research in Ontario in a number of priority areas. CCRM will provide the follow-through on any discoveries ready to be commercialized. Learn more on page 18.

We are also happy to report that we've made substantial progress developing our new companies, which you can read about on page 16, and building our good manufacturing practices (GMP) facility with our partners at University Health Network, MaRS Discovery District and the Province of Ontario.

As well, we've established new collaborations to increase our access to international intellectual property and our industry consortium has grown significantly: from 29 members at the end of 2013 to 44 as we go to print! The growth of our associate membership category has been tremendous and we look forward to engaging in a meaningful way with all of our partners.

In the next five years, we anticipate that CCRM's workforce will triple, we'll spin out more companies that will have bundled Canadian and international technologies at their core, we will develop investment vehicles to fund these promising companies, and Ontario will be a leader in advanced manufacturing of cellular therapies. Momentum is growing in the regenerative medicine industry and CCRM intends to capitalize on the mood and the venture capital that's ready to be invested. We hope that you'll be along for the ride.



Michael May
President and CEO

Greg Bonfiglio
Chair, Board of Directors

Peter Zandstra
Chief Scientific Officer

Michael May
President and CEO
Centre for Commercialization
of Regenerative Medicine

Greg Bonfiglio
Chair, Board of Directors
Centre for Commercialization
of Regenerative Medicine

Peter Zandstra
Chief Scientific Officer
Centre for Commercialization
of Regenerative Medicine

OUR GLOBAL PARTNERSHIPS: BUILDING CONNECTIONS

CCRM is located in Toronto, but its vision extends beyond Canada's coasts. To truly be a global nexus for the regenerative medicine community, we have forged partnerships with stakeholders around the world.

LEGEND

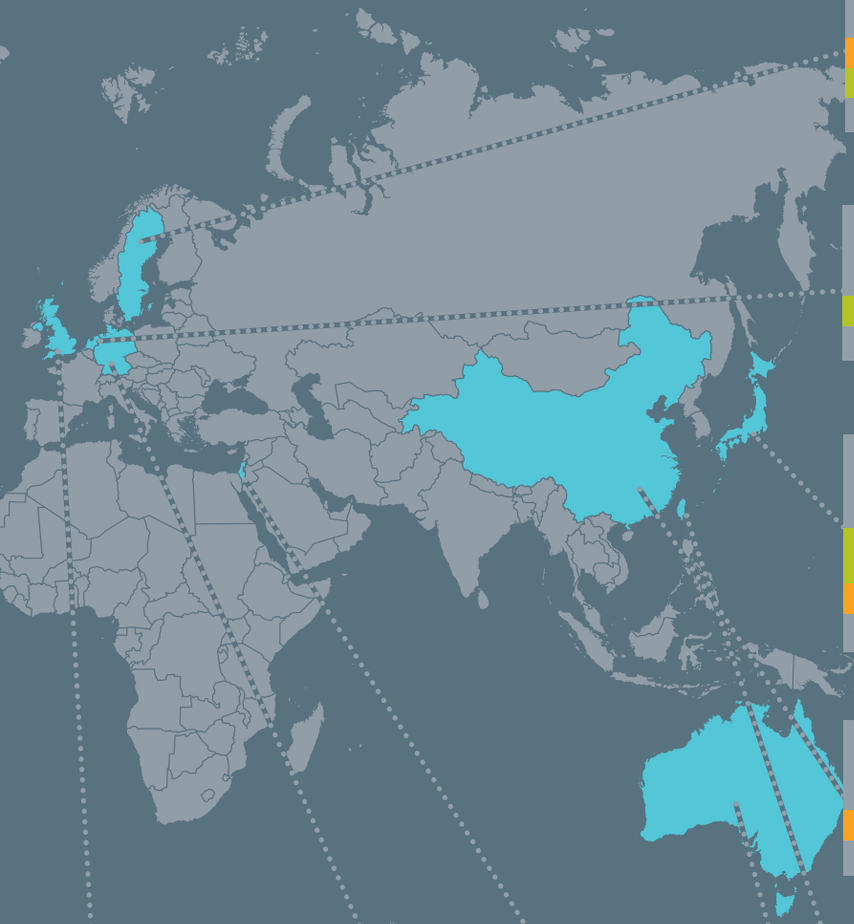
- INDUSTRY CONSORTIUM
- INSTITUTIONAL MEMBERS
- PARTNERS

CANADA

Actium	Toronto, ON
Aspect Biosystems	Vancouver, BC
Axcelon Biopolymers Corporation	London, ON
CellScale	Waterloo, ON
Feldan	Quebec City, QC
Insception Lifebank	Mississauga, ON
Interface Biologics	Toronto, ON
Northern Therapeutics, Inc.	Montreal, QC
Octane	Kingston, ON
Pfizer Canada	Kirkland, QC
RepliCel	Vancouver, BC
Sernova Corp	London, ON
STEMCELL Technologies	Vancouver, BC
Tissue Regeneration Therapeutics	Toronto, ON
Hospital for Sick Children Research Institute	Toronto, ON
McMaster University	Hamilton, ON
Mount Sinai Hospital Lunenfeld-Tanenbaum Research Institute	Toronto, ON
Ottawa Hospital Research Institute	Ottawa, ON
University Health Network	Toronto, ON
University of Toronto	Toronto, ON
Accel-RX	Vancouver, BC
MaRS Discovery District	Toronto, ON
MaRS Innovation	Toronto, ON
McEwen Centre for Regenerative Medicine	Toronto, ON
McGill's Centre of Genomics and Policy (CGP)	Montreal, QC
Ontario Institute for Regenerative Medicine	Toronto, ON
Stem Cell Network	Ottawa, ON
University of Alberta	Edmonton, AB

UNITED STATES OF AMERICA

Agilent Technologies	Santa Clara, CA
Amgen	Thousand Oaks, CA
Athersys	Cleveland, OH
BD	Franklin Lakes, NJ
Biocision	San Rafael, CA
BioLife Solutions	Bothell, WA
Blood Centers of America	West Warwick, RI
Cellular Dynamics International	Madison, WI
Compass Biomedical	Cleveland, OH
EMD Milipore	Auburn, MA
GE Healthcare	Westborough, MA
Janssen Inc.	Raritan, NJ
Lonza	Walkersville, MD
PALL Corp.	Port Washington, NY
Pfizer	Norwich, CT
Progenitor Cell Therapy	Allendale, NJ
Primorigen Biosciences	Madison, WI
SynGen	Sacramento, CA
Thermo Fisher Scientific	Waltham, MA
VistaGen	San Francisco, CA
Alliance for Regenerative Medicine	Washington, DC
California Institute for Regenerative Medicine	San Francisco, CA
Case Western Reserve University	Cleveland, OH
Georgia Institute of Technology	Atlanta, GA
Harvard Stem Cell Institute	Boston, MA



SWEDEN

Karolinska Institute	Solna
Spiber	Stockholm

NETHERLANDS

Applikon Biotechnology	Delft

JAPAN

MEDINET	Yokohama
Nikon	Tokyo
360ip Japan	Tokyo

CHINA

Peking University	Beijing

TAIWAN

Steminent Biotherapeutics	Taipei

GERMANY

Miltenyi Biotec	Bergisch Gladbach
Sartorius Stedim Biotech	Göttingen

AUSTRALIA

Invetech	Melbourne
Stem Cells Australia	Melbourne

UNITED KINGDOM

GE Healthcare	Whitechurch, Cardiff, Wales
Neusentis	Cambridge, England
Sistemic	Glasgow, Scotland
Cell Therapy Catapult	London, England

ISRAEL

Hemostemix	Ness Ziona

BRAZIL

FK Biotec	Porto Alegre

JANUARY TO DECEMBER 2014, NOTABLE EVENTS:

A YEAR OF ACHIEVEMENTS

WELCOME TO CCRM!

APRIL 11 – Corporate leaders, eminent scientists, senior university administrators and others regularly visit CCRM to discuss collaborations or to tour our development facility. Politicians also enjoy seeing what we do. We were delighted to welcome Industry Canada's John Knubley, Deputy Minister, and Anne Cuerrier, Ontario Region Executive Director, to our office and lab.

COLLABORATING DOWN UNDER

JUNE 16 – Exploring the potential of stem cells and their regenerative capacity has been a core focus of medical research in Canada and Australia for many years. As such, CCRM and the Stem Cells Australia consortium have entered into a Memorandum of Understanding to work together to increase the translation of stem cell research in both countries. CCRM representatives visited Australia in November to discuss projects and progress.

PUBLIC SYMPOSIUM A HIT

JUNE 17 – As stem cell researchers from around the world descended on Vancouver to attend ISSCR's annual conference, CCRM and the Stem Cell Network hosted a free event for the public to kick things off. Lawyer and author Timothy Caulfield, UBC professor Kelly McNagny, VC Greg Bonfiglio (Chair of CCRM's Board of Directors) and patient Jennifer Molson discussed the research, regulatory, advocacy and media forces that shape our perceptions about stem cell therapies. Pamela Fayerman, medical/health issues reporter for the *Vancouver Sun*, acted as moderator.

NEW HEALTH SCIENCES ACCELERATOR LAUNCHES

JUNE 25 – Five leading Centres of Excellence for Commercialization and Research (CECR), including CCRM, are working together under the direction of Accel-Rx to develop start-ups into successful health sciences companies in Canada. On August 25th, Accel-Rx was awarded $14.5 million in CECR funding by the Networks of Centres of Excellence.

BIGGER AND BETTER

JULY 25 – Our industry consortium has been growing steadily since we launched. In the summer, we announced that 13 companies – 17 as we go to print – had joined the consortium as associate members. Associate members have access to specific benefits and, after one year, they have the opportunity to become full members. See the full list of 44 members on our website.

"THERAPIES OF THE STATE"

AUGUST – *Nature Biotechnology* examined three government-funded translation centres in three different countries to assess their unique strengths and accomplishments. Canada's CCRM holds its own with the much more amply funded California Institute for Regenerative Medicine and the UK's Cell Therapy Catapult. A copy is available on our website (Media Centre).

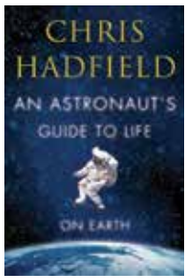
AWARDS

AUGUST 18 - CCRM has been flexing its creative muscles this year and people are noticing. An in-house video – Cell Culture with Bioreactors – created for CCRM's RM video channel (see our website) "wowed" Dr. Paul Knoepfler who ran a stem cell video contest on his popular blog. (At the tail end of 2013, he chose *Signals* as Stem Cell Blog of the Year.) As shown above, the International Association of Business Communicators (IABC) likes our blog too.

With local partners and government support, CCRM is building a state-of-the-art good manufacturing practices (GMP) facility to support both academic and industry clinical trials. It will include a 1,850 sq metre space of clean rooms, quality control labs, support space and offices.

BRANCHING INTO NEW AREAS

SEPTEMBER – So it won't make the *New York Times* best sellers list, but we're pretty proud of our first fling with eBook publishing. With a focus on advancing commercialization of stem cell research and cell manufacturing, we are pleased to share key scholarly articles, perspectives and industry reports, plus a few extras. Download your free copy on our website.



STELLAR INTERVIEW

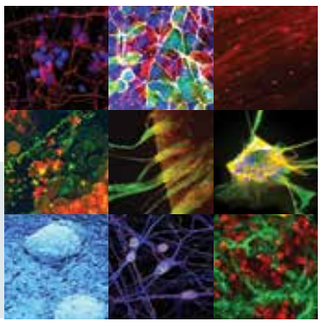
SEPTEMBER 5 – CEO Michael May does a lot of interviews, but meeting Astronaut Chris Hadfield, on CBC's *The Current*, was a highlight. Guest host Hadfield featured stem cell research in his science special. CSO Peter Zandstra was also part of the radio show as he provided an audio tour of CCRM's development facility. (Both fans had Cmdr. Hadfield autograph copies of his book for them, above.) The podcast can be downloaded from our website in the Media Centre.

LIFE ENHANCING DISCOVERY

SEPTEMBER 18 – Principal Investigators Guy Sauvageau (University of Montreal), Peter Zandstra (CCRM, University of Toronto) and Norman Iscove (UHN, University of Toronto), published a study in *Science* announcing their discovery of a new method for the *ex vivo* expansion of human cord blood cells that could potentially increase the number of blood stem cells available for use in the clinic. Please turn to page 16 for more details.

THE WONDROUS WORLD OF STEM CELLS

SEPTEMBER 25 – An interactive, touring science exhibit opened in Quebec to educate families about the wonder of stem cells. Super Cells: The Power of Stem Cells was produced by the Stem Cell Network and the Musée Nature Sciences Sherbrooke, with support from the California Institute for Regenerative Medicine (CIRM), Catapult Cell Therapy and CCRM, and contributions by EuroStemCell. Check for a location near you.



CCRM's first Cells I See art contest attracted more than 1,200 likes on its Facebook Gallery for 20 entries.

A GOLD MEDAL EFFORT

OCTOBER 2 - A large group turned out for CCRM's first Café Scientifique to explore the premise of using stem cells and regenerative medicine to enhance humans. International Olympic Committee executive and former Olympian Richard Pound, lawyer and ethicist Bartha Knoppers, and neuroscientist and author Paul Zehr took the stage with CCRM's Peter Zandstra acting as MC. Follow the tweets at #CCRMCAFE. There was great coverage in the Postmedia Network papers and elsewhere – see our website.

NEW STEM CELL STRATEGY ANNOUNCED FOR CANADIANS

OCTOBER 29 – A coalition led by the Canadian Stem Cell Foundation, and including CCRM, has asked private investors and the Canadian government to fund the development of ten therapies to move into clinical trials in the next ten years. CCRM is the commercialization partner for "Following Through: Realizing the Promise of Stem Cells – A Canadian Stem Cell Strategy & Action Plan (2015-2025)" and estimates 12,000 new jobs and new companies will result from these therapies.

NEW INSTITUTE LAUNCHES

NOVEMBER 25 – CCRM and the Ontario Stem Cell Initiative have established the Ontario Institute for Regenerative Medicine (OIRM) – a research, development and commercialization institute dedicated to the translation of stem cell research into curative therapies for major degenerative diseases. OIRM is funded by the Government of Ontario. This is wonderful news for Ontario's RM community. See page 18 for more.

OPENING DOORS FOR RESEARCHERS

When asked whether he has benefited from working with CCRM, Dr. Gordon Keller – Director of the McEwen Centre for Regenerative Medicine, Professor at the University of Toronto and Senior Scientist at the Ontario Cancer Institute – replies with an enthusiastic "yes!"

He mentions how CCRM has opened doors for him with Big Pharma and biotech companies, but one assumes that his reputation alone might get him meetings. He explains that CCRM's strong ties to industry have proven helpful in a number of ways, from establishing collaborative partnerships to appreciating how his work can be tailored to suit industry's needs, especially in the areas of predictive toxicology and metabolism studies with the hepatocyte (liver) cells his lab generates.

It is a huge advantage for industry to be able to safely and accurately predict how patients will respond to certain compounds before spending millions on clinical trials that could fail. Primary hepatocyte toxicology studies offer a well-established model for testing potential therapeutics.

In addition to funding that Dr. Keller received from the McEwen Centre-CCRM Commercialization Impact Prize, he says he has also benefited from work conducted in CCRM's development facility to establish large-scale manufacturing of pluripotent stem cell-derived cardiomyocytes, which are being utilized in a three-dimensional cardiac microtissue to investigate the mechanism of action on compounds that affect contractility (an unmet need in drug discovery).

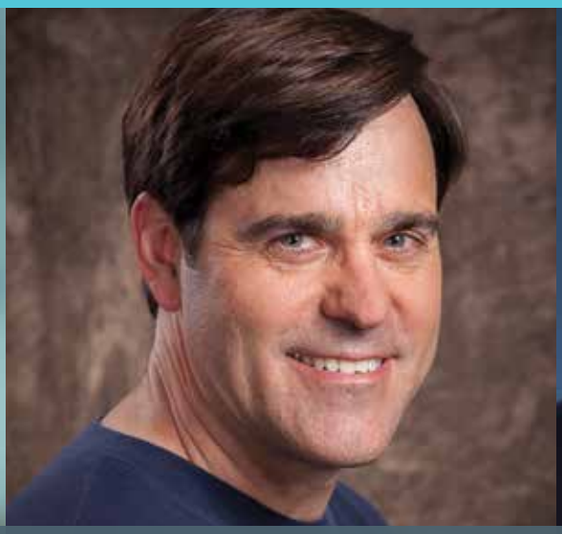
Looking at the bigger picture, Dr. Keller says he thinks it was "forward thinking [of the Networks of Centres of Excellence] to fund a commercialization centre in regenerative medicine that is focused on identifying real-world applications for the work we [scientists] are doing. I would encourage much more funding in this area."

Gordon Keller, *PhD*
McEwen Centre for Regenerative Medicine, University Health Network

TRAILBLAZERS AND MENTORS

They are a Who's Who of experts and they have generously donated their time and knowledge to help establish and advance the activities of CCRM. These 11 scientists and engineers are steps away from CCRM's office and serve on our Founders Advisory Board.

Mick Bhatia
McMaster Stem Cell and Cancer Research Institute, Hamilton



James Ellis
The Hospital for Sick Children Research Institute, Toronto

Gordon Keller
McEwen Centre for Regenerative Medicine, University Health Network, Toronto

Molly Shoichet
Institute of Biomaterials & Biomedical Engineering, University of Toronto

William L. Stanford
Ottawa Hospital Research Institute

Peter Zandstra
CCRM
University of Toronto

Janet Rossant, Chief of Research at the Hospital for Sick Children, Director of the Ontario Institute for Regenerative Medicine and out-going President of the International Society for Stem Cell Research, is one of the most cited stem cell researchers in the world.

Back in 2005, **Andras Nagy** established the first human embryonic stem cell line. He followed up this achievement with another remarkable breakthrough in 2009: he discovered a new non-viral method of creating induced pluripotent stem cells that could lead to possible cures for many devastating and chronic diseases. In 2014, he discovered a new class of stem cells: F class. In addition to receiving many impressive honours and awards, **Mick Bhatia**'s *Nature* paper, that demonstrated that adult cells could be reprogrammed directly to blood cells, was described as a "world-beating breakthrough" by the *Toronto Star* in 2010.

Michael Rudnicki, an Officer of the Order of Canada, is an Associate Editor of two high impact factor publications: *Journal of Cell Biology* and *Cell Stem Cell*. His lab identified Pax7 as a transcription factor required for the specification of satellite cells, and identified Wnt signaling as playing an important role in muscle stem cell function. Another prolific award winner, **Molly Shoichet** was elected to the Canadian Academy of Sciences in 2008, the highest distinction that a Canadian scientist can receive. She's a polymer expert who advises CCRM's biomaterials scientists and she has founded two spin-off companies from research originating in her lab.

The achievements of the remaining members are equally impressive and too numerous to mention. CCRM is grateful for everyone's counsel and support. Please see CCRM's website for everyone's biographical details.

Janet Rossant
The Hospital for Sick Children Research Institute, Toronto

Andras Nagy
Lunenfeld-Tanenbaum Research Institute, Mount Sinai Hospital, Toronto

Armand Keating
Princess Margaret Hospital, University Health Network, Toronto

Michael Rudnicki
Ottawa Hospital Research Institute

Paul Santerre
Institute of Biomaterials & Biomedical Engineering, University of Toronto

Lee Buckler,
VP of Business and Corporate Development
RepliCel Life Sciences Inc.

DELIVERING ON A PROMISE

CCRM has a fan in Vancouver-based RepliCel Life Sciences Inc., a clinical stage regenerative medicine company focused on the development of autologous cell therapies.

Lee Buckler, VP of Business and Corporate Development, believes that "a cell therapy industry has emerged in Canada in the last year or so that never existed previously. CCRM has changed that. Not single handedly, but it is in large part due to the impact CCRM has played. [CCRM] has put Canada on the global map." Mr. Buckler says it's incumbent on us to deliver on projects already initiated and the strategic promises we've made for the next five years of operations; however, "[CCRM] has already made an important contribution."

At the very least, we've made a meaningful contribution to RepliCel™. It joined our industry consortium in August 2014 and we've already been able to offer valuable assistance in the form of regulatory support in an application to Health Canada for a Phase 1/2 clinical trial to treat patients suffering from chronic Achilles tendinosis. The "ReaCT" study, as it's known, received a No Objection Letter and the study is now before the University of British Columbia's Clinical Ethics Review Board as the next step to have "ReaCT" begin enrolling patients at the University of British Columbia Hospital.

RepliCel's next collaboration with CCRM will be a process development project to address how to optimize a critical step in its bioprocessing. And, amazingly, the company already has its next projects with CCRM identified. RepliCel intends to transfer its manufacturing from Europe to North America – ideally to a facility in Canada managed by CCRM. This is dependent upon our ability to develop advanced cell therapy manufacturing capabilities in the country, a priority for us right now and something that is progressing well.

Mr. Buckler, a proud Canadian, is eager to see Canada lead the cell therapy industry. We promise to do our best to make that happen.

OUR PEOPLE:

SHARING EXPERTISE

A Way with People

Rahul Sarugaser
PhD, MBA – Director, Business Development

Rahul is our resident over-achiever. With a PhD in Biomedical Engineering from the University of Toronto, an MBA from Oxford University and an out-going personality, he is the ideal person to lead CCRM's business development activities. His team has grown our industry network to over 100 Canadian and international companies, and is now building CCRM's network of investment partners.

A Talented Multi-tasker

Elizabeth (Liz) Csaszar
PhD – Development Scientist, Cell Manufacturing

Liz is a woman of many talents. In addition to her research that focuses on development, scale-up and clinical translation of blood cell technologies, she has starred in a CCRM-produced bioreactors video, co-authored significant papers published in *Cell Stem Cell, Science* and *Blood* and she is the 2013 winner of the Donnelly Thesis Prize from the University of Toronto.

A Leader Worth Following

Sowmya Viswanathan
PhD – Director Regulatory and Clinical Translation

Until recently, Sowmya has been consulting for CCRM in the area of regulatory and clinical translation and co-leading our popular Cellular Therapies Manufacturing and Clinical Trials in Canada workshop with CCRM's Nick Timmins. We're excited to have her expertise at CCRM as we embark on running a new GMP cell therapy facility at MaRS, in partnership with University Health Network.

A Head for Numbers

Michael Israels
Chief Operating Officer/Chief Financial Officer

Michael joined CCRM in late 2013 as the COO, but with a strong investment background and a hole to fill, he took on additional duties as CFO in 2014. With executive management and advisory experience earned at several investment companies, Michael is perfectly poised to lead CCRM's efforts to connect with venture capital and other investors to support CCRM's company creation activities.

COMPANY CREATIONS:

MOVING DISCOVERIES FORWARD

In last year's report, we identified seven promising technologies, of which five were approved to progress to the company creation stage. These five have been the focus of considerable time and resources over the past year and we continue to review each one for its feasibility and likelihood of success. At present, we are concentrating on three highly promising initiatives that you can read about here.

EXCELLTHERA

Developed with two key technologies by Professors Peter Zandstra (University of Toronto, Institute of Biomaterials and Biomedical Engineering) and Guy Sauvageau (Université de Montréal) and support from IRICoR (Institute for Research in Immunology and Cancer – Commercialization of Research), ExCellThera will offer a best-in-class process to boost the number of stem cells available to patients undergoing transplants for leukemias and to create a pipeline of additional blood products to address further unmet clinical needs.

A new molecule, discovered by Dr. Sauvageau, that allows for the multiplication of stem cells required for clinical-grade umbilical cord blood (UCB) units, is being combined with Dr. Zandstra's Fed-Batch technology – a new type of bioreactor developed for culturing cells. Together, the number of UCB units available for a transplant in humans can be multiplied significantly. More stem cells mean a better match for donors and a more successful transplant. ExCellThera's process is designed to be adaptively scaled-up for human transplantations in clinical settings.

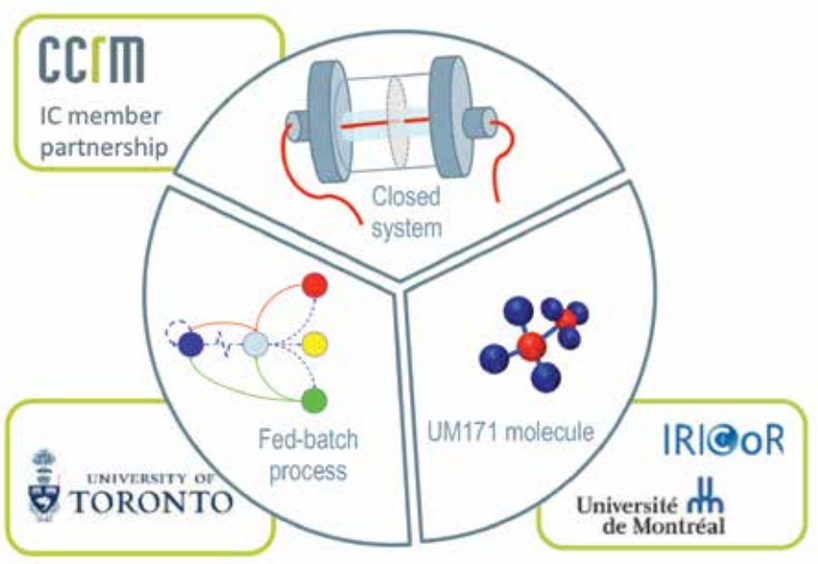
ExCellThera has two products in development:

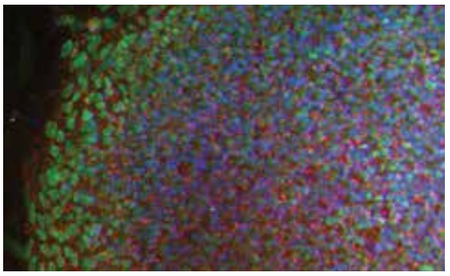
Primary: *Ex vivo* expanded CD34+ cells for use in hematopoietic stem cell transplantation (HSCT) for hematologic/immune indications, with a primary focus on acute myeloid leukemia (AML); and,

Secondary: 1. Gene therapy products enabled by enhanced gene transfer capabilities and *ex vivo* expansion; 2. Mature blood cell products enabled by increased pre-expansion of primitive CD34+ cells.

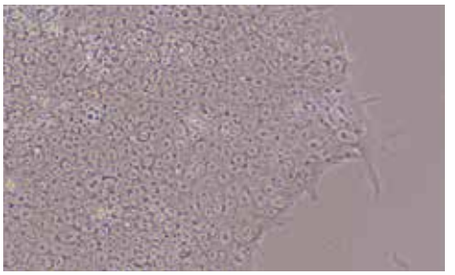
Guy Sauvageau

Peter Zandstra

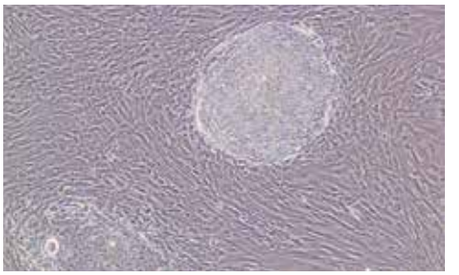




iPSC line derived from patient with cystic fibrosis. Green, NANOG; Red, SSEA4.



iPSC lines derived using non-integrating episomes in a feeder free system.



Newly formed iPSC colony derived using non-integrating episomes.

"IPSC SERVICE CO."

Already in operation, iPSC Service Co. performs reprogramming of patient-derived somatic cells to induced pluripotent stem cells (iPSCs) for academic researchers and clinicians on a cost-recovery basis.

Taking place in CCRM's development facility, CCRM scientists and technicians specialize in non-integrative strategies and routinely reprogram both fibroblasts and cells isolated from peripheral or cord blood samples.

Additional services include advising on all aspects of the reprogramming process, from explaining sample collection options to training and guidance during pluripotent culture of the delivered iPSCs.

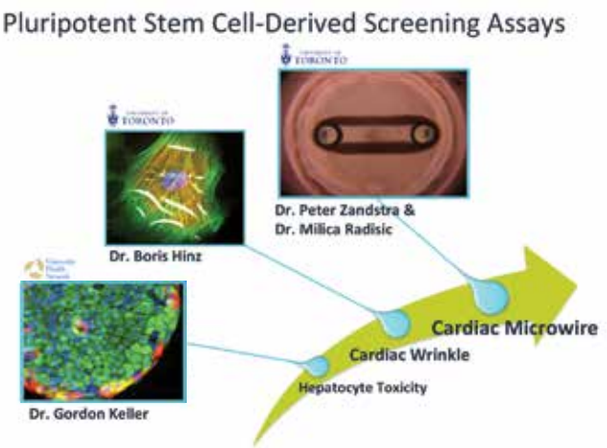
In keeping with CCRM's goal to advance regenerative medicine efforts in Canada, we strive to support the needs of clinicians and their patients by customizing our deliverables according to their needs. We have worked with difficult to reprogram patient cells as well as peripheral blood samples as small as 1 mL in volume.

To date, CCRM has produced and delivered over 50 iPSC lines that are being used for disease modeling and drug screening in institutions across Canada. We have derived two control iPSC lines that can be distributed under material transfer agreements. Our future plans include expanding our capabilities to include additional reprogramming technologies, gene correction of iPSCs and differentiation of iPSCs.

"SCREEN CO."

The development of new drugs is very expensive, costing between $500 million and USD $2 billion per FDA-approved new chemical entity (NCE). While the time and cost of developing new drugs has been steadily increasing over the last ten years, the number of approved NCEs has not. Of those drugs that are approved, only about one third recover their total development costs. There is a market for technologies capable of increasing the cost-effectiveness and efficiency of NCE development pipelines and CCRM is developing disease and patient-specific models for testing purposes to meet this need.

Screen Co. will bundle screening platform technologies from our academic partners, Professors Peter Zandstra, Milica Radisic and Boris Hinz (University of Toronto) and Gordon Keller (University Health Network), and operate as a service company to generate multiple sets of validation data to test NCEs before they enter costly clinical trials. In the future, Screen Co. will pursue CCRM's drug discovery in collaboration with a pharmaceutical partner.



Screening Platforms: Cardiac (efficacy) and Hepatic (toxicity)

Screen Co. has a deep pipeline of induced pluripotent stem cell (iPSC)-derived screening assays, with the cardio microwire (CMW) being the most mature asset. The CMW is an *in vitro* test that measures the force of contraction of 3D human pluripotent stem cell (hPSC)-derived microtissues that can mimic the properties of adult tissues. It, along with the higher-throughput Wrinkle technology - an *in vitro* assay composed of a thin polymer surface modification that is applied to cell culture plates to measure 'wrinkles' under cell force exertion in a high throughput screen (HTS) – are well positioned to test respiratory and musculoskeletal conditions.

A second screening platform consists of 3D hepatic organoids that have similar functionality and CYP enzyme expression to the adult human liver and will be used for toxicity testing.

ONTARIO INSTITUTE FOR REGENERATIVE MEDICINE:

BUILDING ONTARIO'S STRENGTH IN REGENERATIVE MEDICINE

CCRM and the Ontario Stem Cell Initiative (OSCI) have worked together closely since we both launched in 2011. We share a similar vision for how the regenerative medicine field should progress in Canada and it includes supporting advances that will address the staggering costs of chronic and degenerative diseases affecting the country's aging population.

Our solution has been to establish an institute for regenerative medicine that will focus on research, development and commercialization to translate stem cell research into curative therapies for cardiovascular disease, diabetes, blindness, lung disease, neurodegenerative disorders and blood and musculoskeletal diseases.

This year, we achieved our goal. On November 25th, Reza Moridi, Minister of Research and Innovation, announced the launch of the Ontario Institute for Regenerative Medicine (OIRM) – a partnership between CCRM and OSCI, with $3.1 million in funding provided by the Province of Ontario.

OIRM will bring together foundational research, education, policy, entrepreneurship, cell manufacturing, screening and clinical trials to develop curative therapies for specific disease areas. OIRM's institutional partners include McMaster University, University of Ottawa, University of Toronto and Western University and its affiliated research hospitals.

OIRM has already funded the following:

DISEASE TEAM GRANT AWARDS
$250,000 each

Gordon Keller,
University Health Network
Fixing damaged hearts with heart muscle derived from stem cells

Valerie Wallace,
University Health Network
Restoring vision in macular degeneration through stem cell therapies

Mick Bhatia,
McMaster University
Developing novel stem cell strategies for immunotherapy

NEW IDEA GRANT AWARDS
$75,000 each

Rama Khokha,
University Health Network
Production of a synthetic environment for liver stem cell expansion

James Ellis,
SickKids Research Institute
Control of RNA translation into proteins in human stem cells, neurons and disease

Peter Zandstra,
University of Toronto
Engineering a functional human thymus from pluripotent stem cells

Duncan Stewart,
Ottawa Hospital Research Institute
Role of short RNA fragments in mediating the anti-inflammatory effects of bone marrow stem cells in sepsis

John Coles,
SickKids Research Institute
A personalized approach to correction of genetic heart defects.

Disease Team Grant Winners

New Idea Grant Winners


Minister Reza Moridi and Michael May

TRAINING AND EDUCATION:

TEACHING, SHARING, EXPLORING

CCRM's training and education programs connect the regenerative medicine community to a variety of professional development and networking opportunities to provide its members with the necessary tools to drive technologies from the bench to the bedside.

CCRM's educational initiatives bring together trainees, academics and professionals to foster continual discovery, collaboration and skills-building. When we're not hosting our own events, you can find us supporting our partners with their education programs. That's because we know how important collaboration and knowledge sharing are to realizing the community's goals. Here's a sample of some of our events.

THE BUSINESS OF REGENERATIVE MEDICINE: NEW THERAPIES, NEW MODELS

With the teacher to student ratio high, this course provided a unique opportunity for those establishing their careers to learn from and network with industry and academic leaders in the community. Dr. Arnold Caplan (Case Western Reserve University) passed the baton to CCRM to host the course in Toronto, to great reviews.

TILL & MCCULLOCH MEETINGS

Produced in partnership with the Stem Cell Network, this year's Till & McCulloch Meetings welcomed over 450 stem cell scientists, bioengineers and industry representatives to Ottawa, Ontario for Canada's premier stem cell event. They heard from international speakers and Canada's most distinguished experts on the state of the field.

CELLULAR THERAPIES MANUFACTURING AND CLINICAL TRIALS IN CANADA WORKSHOP

This workshop brought together academic and industry representatives to focus on downstream processing and manufacturing challenges. Through lectures, case studies and demonstrations of some of the latest manufacturing technologies, students gained an appreciation of the complexities of conducting clinical trials in Canada.

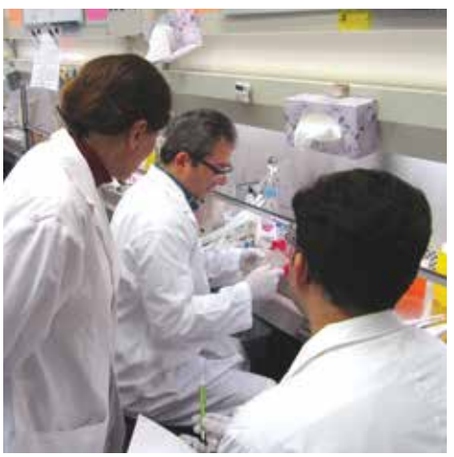
HUMAN INDUCED PLURIPOTENT STEM CELL WORKSHOP

Producing stem cell lines is vital to many research projects in the community. This workshop provided participants with hands-on lab time, lectures from leading academics and industry professionals, and networking opportunities, imparting them with the skills and theoretical background necessary to produce high quality stem cell lines.

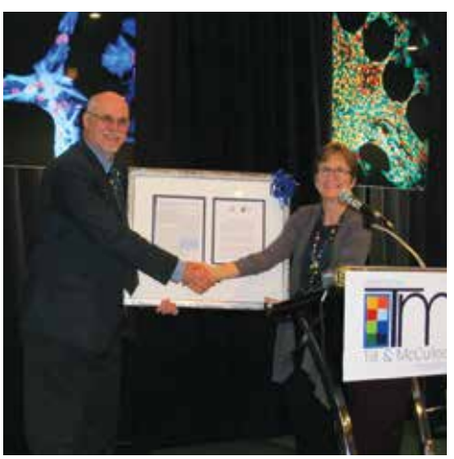
COMMUNICATIONS AND COMMERCIALIZATION FOR SCIENTISTS WORKSHOP

The Stem Cell Network produces soft skills workshops for trainees in conjunction with major Canadian stem cell events. CCRM helped to develop the program, provided content and participated in the workshop prior to ISSCR's 12th Annual Meeting. Trainees left with the skills to successfully communicate their research projects to media and industry.

RM business course



HiPSC workshop



Till & McCulloch Meetings 2014

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Lunenfeld-Tanenbaum Research Institute
MOUNT SINAI HOSPITAL
Joseph and Wolf Lebovic Health Complex

SickKids

UHN
Toronto General
Toronto Western
Princess Margaret
Toronto Rehab
COURAGE LIVES HERE

UNIVERSITY OF TORONTO

Founding Partners

CGP Centre of Genomics and Policy
Centre de génomique et politiques

MaRS

MaRS Innovation

McEWEN CENTRE FOR REGENERATIVE MEDICINE

oirm
Ontario Institute for Regenerative Medicine

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