2016 ANNUAL REPORT

BUILD. ADVANCE. BRIDGE.

DELIVERING BUSINESS AND SCIENTIFIC EXPERTISE

CCRM

Commercializing
Living Therapies

CCRM is a Canadian, not-for-profit, public-private consortium supporting the development of foundational technologies that accelerate the commercialization of cell and gene therapies, and regenerative medicine-based products and technologies.

These therapies and technologies have the potential to transform health care, with the promise to treat, manage and perhaps cure some of the most debilitating and costly diseases in the world today. Regenerative medicine aims to harness the power of stem cells, biomaterials and molecules to repair, regenerate or replace diseased cells, tissues and organs. However, many new and potentially life-changing cell, gene and regenerative medicine-based treatments never reach patients because they are not successfully moved from the laboratory to the clinic.

In order to fulfill the promise of regenerative medicine and cell and gene therapies to treat the many diseases affecting our population, a world-renowned group of stem cell scientists and bioengineers have come together to form CCRM.

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CCRM

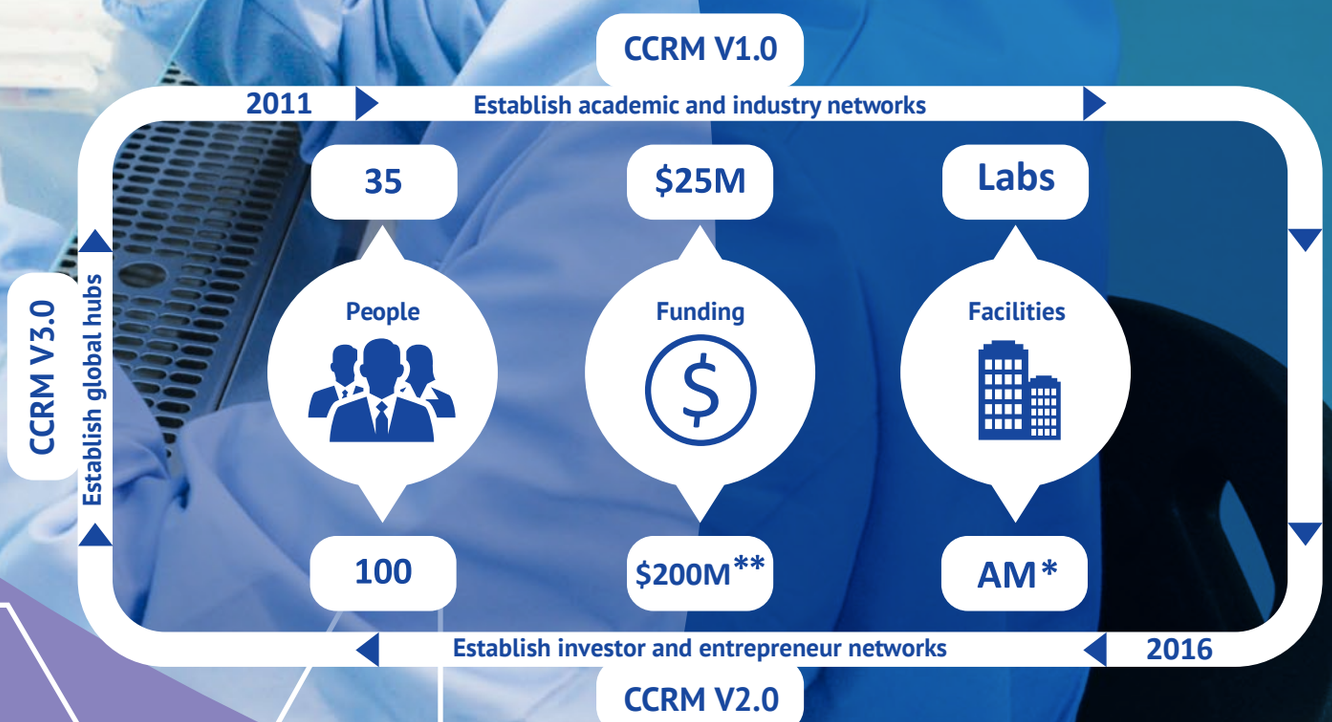
OUR MISSION

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 generate sustainable health and economic benefits through global collaboration in cell and gene therapy, and regenerative medicine.

OUR VISION

To be the preferred global destination for the best people, technologies, clinical trials, companies and investments in regenerative medicine.

To be the premier global enabler of clinically-tested, revolutionary new medical therapies and foundational technologies.



* AM - MaRS facility includes advanced manufacturing facility

** $200M includes regenerative medicine-focused investment vehicles

REACHING CRITICAL **SCALE-UP MILESTONES**

Photo, L-R: Peter Zandstra, Michael May, Greg Bonfiglio

Quite by accident, our two biggest news stories in 2016 kicked off the year and capped it. In January, Prime Minister Justin Trudeau and GE Healthcare made the public announcement that they were each committing $20 million to CCRM to establish a centre for advanced therapeutic cell technologies (CATCT). Then in December, Bayer and Versant Ventures made a momentous investment to establish BlueRock Therapeutics. You can read more about this and CCRM's role on page 18.

Between January and December, here are a few other things we accomplished.

In an effort to get CATCT (which we refer to as BridGE@CCRM) running and open for business, we doubled our headcount (from approximately 30 to 60 staff), commissioned a lab at the Princess Margaret Cancer Research Tower to serve as CATCT's temporary home base, installed one of the largest bioreactor development suites in North America, created new standard operating procedures, started eight internal projects and multiple technical evaluations, and partnered with cell therapy companies.

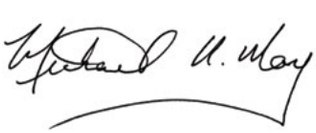
In tandem with the activity in BridGE, CCRM has been continuing to build its other services. While our development facility has been offering contract services for several years now in cell reprogramming and engineering – just to name one area – our business development group has been doing some hiring of its own. For example, we now have a regulatory and clinical affairs expert who can advise and assist clients in planning for and preparing technical documents and regulatory forms for submission to Health Canada. In the pages to follow, we think you will find many reasons to deepen your engagement with CCRM.

In the Highlights section (page 6), you will read about our success in solidifying our global collaborations, culminating in the exciting news that CCRM Australia is now operating in Melbourne.

We also rebranded in the spring, with a "new" name – we go by CCRM only now except in legal documents – a new logo and new corporate colours. The timing reflects our maturation as an organization and a shift from commercializing regenerative medicine-based products and technologies to adding cell and gene therapies into our repertoire.

Finally, we have been busy preparing for our big move. Early in 2017 we will open the doors to our new 40,000 sq. ft./3,700 sq. m. home in the MaRS Discovery District in Toronto. This "stem cell city" will house CATCT and CCRM's development facility where we conduct wet diligence and provide a variety of services for a fee. There will be a good manufacturing practice (GMP) facility that we will operate in partnership with the University Health Network, and offices, meeting rooms and collaborative spaces for CCRM staff and our partners. Medicine by Design and the Ontario Institute for Regenerative Medicine will also be sharing the space to provide a truly collaborative, regenerative medicine-focused environment.

2017 will be another busy year at CCRM. We look forward to engaging with you.



Michael May
President and CEO

Greg Bonfiglio
Chair, Board of Directors

Peter Zandstra
Chief Scientific Officer

CDMO SPECIALISTS

FROM CONCEPT **TO MARKET**

With the Centre for Advanced Therapeutic Cell Technologies (CATCT) underway, a suite of induced pluripotent stem cells and associated services available, and new hires to augment our consulting capabilities, CCRM has evolved into a Contract Development Manufacturing Organization (CDMO) with all the services necessary to move our customers and partners from concept to market. Here's how our new business units are defined.

1

BUILD. The business development team ("Build") has been conducting due diligence on intellectual property (IP) and working with the industry consortium since CCRM's early days, but it has expanded its focus to offer new services in business planning and regulatory consulting. The IP and tech assessments continue.

2

ADVANCE. CCRM's Advance scientists and technologists have expertise that comes from experience. They have generated more than 90 iPSC lines and completed 30 projects. The team offers cell reprogramming and engineering, technology assessments and translation in collaboration with Build, and cell differentiation and protocol development.

3

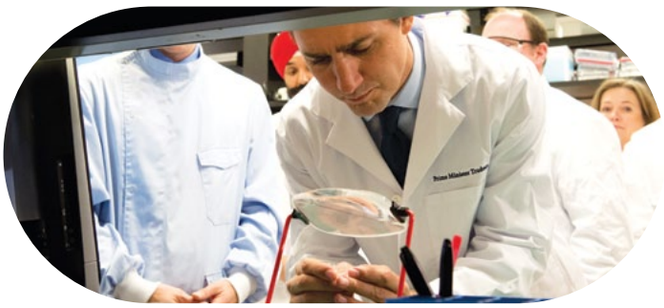
BridGE. With an emphasis on cost and risk analysis, this team is bridging the gap to industrial manufacturing and looking for solutions to technical challenges that impact current and future workflows. Offering scale-up and scale-out, process closure and automation, as well as media development, BridGE has employees from CCRM and GE Healthcare.

4

DELIVER. CCRM is building and will operate a good manufacturing practice (GMP) facility where cells will be manufactured in a sterile environment that adheres to governmental best practices and standard operating procedures, for use in clinical trials. Working with University Health Network, the Deliver team is putting the processes in place to launch this facility.

JANUARY TO DECEMBER 2016

A YEAR OF **HIGHLIGHTS**



JANUARY 13 – Prime Minister Justin Trudeau tours CCRM's development facility before making a public announcement to invited guests and the media about his government's $20 million funding to support the establishment of a centre for advanced therapeutic cell technologies (CATCT). GE Healthcare, our anchor partner in CATCT, also announces its own $20 million commitment.

EXCELLTHERA

FEBRUARY – ExCellThera, a spin-off from CCRM and IRICoR, begins enrolling up to 25 patients for its first Phase I/II clinical trial to test the safety and efficacy of its lead product, ECT-001, for the expansion of hematopoietic stem cells (HSCs) to treat acute myeloid leukemia (AML) and other malignant blood disorders.

APRIL – Beginning with Leiden University Medical Center in the Netherlands, CCRM signs memorandums of understanding (MOUs) with groups in Israel and Japan who are also eager to establish international commercialization platforms in regenerative medicine. With Australia's commitment secured in 2014, the seeds are planted for a global collaboration.

MAY – AVROBIO, one of CCRM's portfolio companies that targets cancer and Fabry disease, is awarded incubator space at the JLABS facility in Toronto and it is nominated for The New Economy Awards' "innovative gene therapy research company of the year 2016." In August, AVROBIO announces it has raised $25 million in a Series A financing with VC partners.

MAY 17 – CCRM is invited to host an entrepreneurial workshop on tissue engineering as part of the World Biomaterials Congress' program. Our speakers, all hailing from industry and many with start-up experience, share anecdotes, tips and strategies on entrepreneurship, much to the delight of the audience.

OCTOBER 24 – CCRM, Stem Cell Network and the Ontario Institute for Regenerative Medicine take the Till & McCulloch Meetings west to Whistler, British Columbia, and the Canadian stem cell community follows. The 2.5 day conference is filled with great talks and posters, lots of networking opportunities, educational and practical workshops and the breathtaking beauty of the mountains.

OCTOBER 26 – Monash University, the Australian Regenerative Medicine Institute and the Commonwealth Scientific and Industrial Research Organisation launch CCRM Australia, a partnership with CCRM to move scientific discoveries and technologies to the market. This builds upon the MOU signed by CCRM and Stem Cells Australia in 2014 to increase the translation of stem cell research between the two countries.

DECEMBER 12 – BlueRock Therapeutics is launched as the result of an investment of $225 million USD from Bayer AG and Versant Ventures. The company will create induced pluripotent stem cell therapies to treat chronic heart failure and other conditions. The cardiac program will leverage intellectual property from Dr. Gordon Keller at UHN, and CCRM will support the manufacturing platform.

Photo credit: Queen's Printer for Ontario, 2016

BY THE NUMBERS:

514,473,368
MEDIA IMPRESSIONS FROM ANNOUNCEMENT OF CATCT (COVERED BY 429 DISTINCT SOURCES, INCLUDING 90 GLOBAL OUTLETS)

18,733
VIEWS OF CATCT NEWS RELEASE ONE DAY AFTER ANNOUNCEMENT

47
ARTICLES (AND TWO PODCASTS) ABOUT CCRM IN 2016

12
BLOGGERS PARTICIPATE IN SIGNALS' FIRST BLOG CARNIVAL

CCRM BRIDGE

CATCT HELPS TO BRIDGE **THE CELL THERAPY COMMERCIALIZATION GAP**

24
BRIDGE STAFF HIRED

8
PROJECTS LAUNCHED

In 2016, the BridGE team has evolved into a primed and ready business unit. Its focus is the Centre for Advanced Therapeutic Cell Technologies (CATCT) and everything required to make it operational and successful. Projects have already begun and the workload will grow when CATCT's permanent space is established in its new home in the MaRS Centre.

CATCT is an advanced manufacturing solutions facility that is addressing challenges in cell and gene therapy (CGT) production. GE Healthcare, the anchor partner, has committed $20 million to the project, matched with $20 million from the Federal Economic Development Agency for Southern Ontario (FedDev Ontario).

CATCT is bridging the gap from preclinical research to commercial-ready manufacturing of CGTs, and 2016 marks the first year of the initiative. To execute on CATCT, CCRM and GE Healthcare have built a team of experts in manufacturing technologies and processes, named BridGE. This team is uniquely positioned to advise clients on the challenges of commercial scale manufacturing of CGTs, and to work with them to identify, develop and adopt suitable solutions.

Since Prime Minister Justin Trudeau announced the CATCT funding in January, CATCT has moved swiftly from concept to reality. The BridGE team is now executing against multiple projects and delivering tangible results. CATCT provides access to leading capabilities for scale-up and scale-out, process closure and automation, process optimization and intensification, risk reduction and media development.

"Supporting clients through contract development work is an opportunity for BridGE to help realize the delivery of therapeutic cells to market, and enhance health outcomes," says Dr. Nick Timmins, VP Technology and Director, BridGE@CCRM.

One example is the scale-up of pluripotent stem cell (PSC) culture in suspension bioreactors. In this project, BridGE is establishing capabilities for industrial and commercial scale and quality manufacturing of PSCs for use as a starting material to produce a variety of therapeutic cells products.

The BridGE team recognizes that in a cell manufacturing workflow, different technologies from multiple companies are required to effectively advance technologies and develop solutions. Furthermore, some solutions do not yet exist, or are inadequate to meet the needs of this emerging industry, which provides a powerful opportunity for the BridGE team to advance new solutions. Solicitations of new tools and technologies are welcome, from proof-of-concept to original equipment manufacturer (OEM) integration.

"For GE, a benefit [for involvement in CATCT] is the potential for collaboration. Engaging and working with other organizations and academics provides a fruitful way for GE to have visibility to new technologies from a cell and gene therapy manufacturing perspective," says Dr. Aaron Dulgar-Tulloch, Director, BridGE@CCRM for GE Healthcare's Cell Therapy Technologies.

The BridGE team champions collaboration, ensuring that the strategic goals of all clients and partners are considered at the outset. The team also appreciates that the advancements that take place in CATCT will benefit the regenerative medicine community more broadly, with the overarching goal of benefiting greater numbers of patients in the future.

Looking forward, the BridGE team welcomes opportunities to help clients address their challenges in CGT manufacturing, and is open to working with partners to advance early-stage industrial manufacturing technologies to maturity.

CCRM › BridGE

ENABLING CLIENTS TO **ADVANCE TECHNOLOGIES AND CELL THERAPY RESEARCH**

CCRM's Advance team specializes in contract services, evaluation of new technologies and early process development for cell manufacturing. With an eye to the future, the Advance team works to push new technologies towards commercialization and clinical translation.

CCRM's Advance scientists and technologists reprogram patient cells to induced pluripotent stem cells (iPSCs), perform gene correction in iPSCs and develop reporter cell lines for academic clients. They evaluate and optimize new regenerative medicine technologies at the bench to support the due diligence activities of the Build team. Consisting of experts in human stem cell biology, the team also performs product testing and early process development of differentiation protocols for clients, while collaborating on the translation of scientific discoveries.

"CCRM often contributes to our research projects beyond the contracted scope of work. As providers of patient-specific induced pluripotent stem cell lines for our cystic fibrosis research, CCRM has shared valuable expertise with the team, benefiting the project overall," says Dr. Christine Bear, The Hospital for Sick Children.

CCRM › ADVANCE

SUPPORTING COMPANIES **IN BUILDING THEIR COMMERCIALIZATION ABILITIES**

CCRM's business development team offers expertise and guidance to its industry and academic partners in business, regulatory and intellectual property (IP) issues. The team's unique blend of commercialization and technology expertise prepares their partners for success as new technologies are developed.

"In the health charity sector, patients care deeply about treatment-oriented research," says Dr. Mary Sunderland, Director of Research and Education, the Foundation Fighting Blindness. "We worked closely with CCRM to develop a unique consultation project focused on understanding the commercialization potential of our current research portfolio. Both the consultation process and the final reports revealed key insights about how to strategically develop our research portfolio to maximize our research impact and ultimately achieve our goal of developing new treatments."

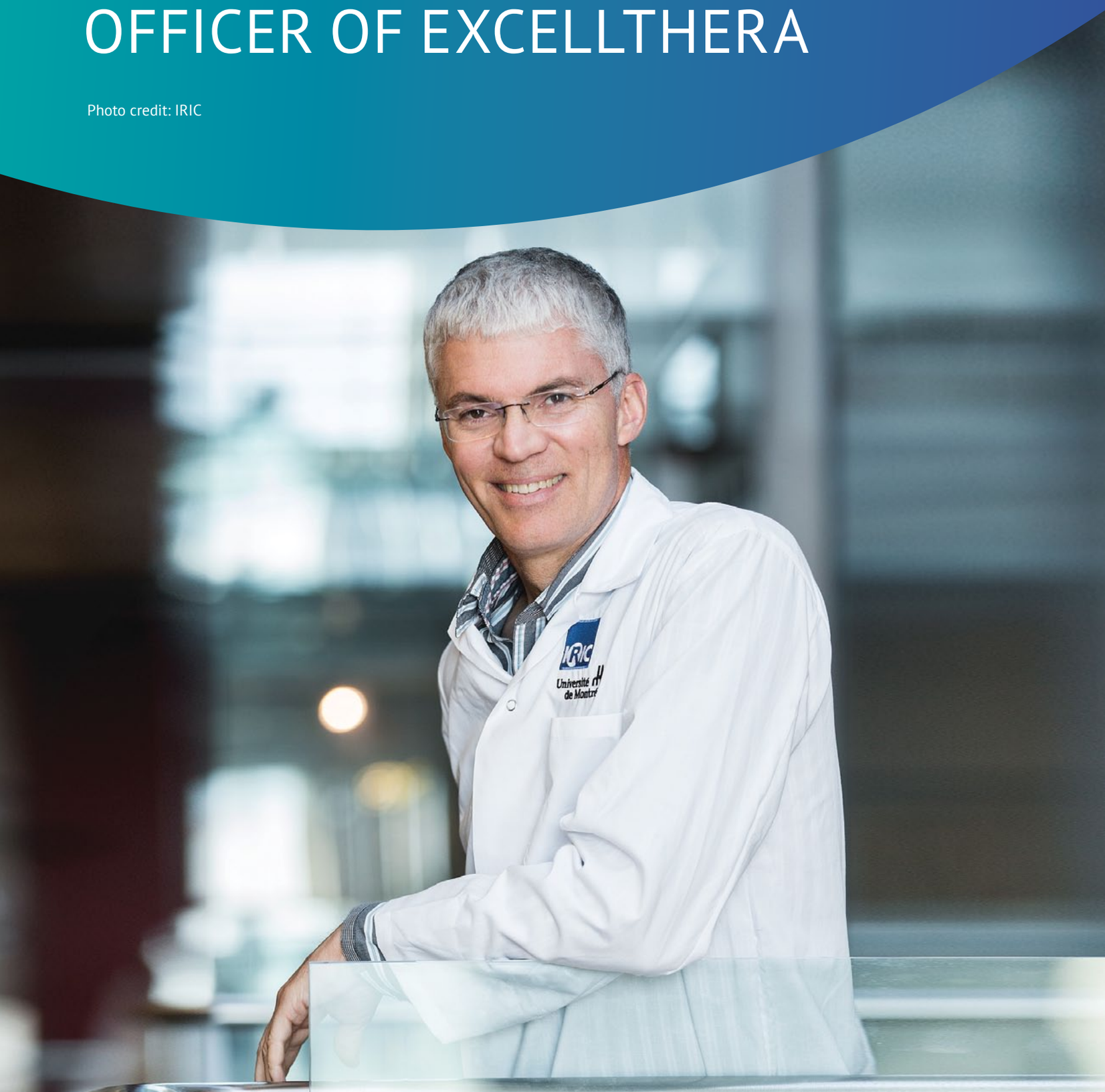
In 2016, the business development team was strengthened with the addition of IP and regulatory affairs experts. To date, the team has delivered on over 1,000 consultation hours and 300 technology reviews.

CCRM BUILD

ACADEMIC PARTNER

GUY SAUVAGEAU, CEO, SCIENTIFIC FOUNDER AND CHIEF SCIENTIFIC OFFICER OF EXCELLTHERA

Photo credit: IRIC



Guy Sauvageau has a deep understanding of how the activity of hematopoietic stem cells (HSCs) is regulated. To convert this knowledge into therapies, Dr. Sauvageau collaborated with two Canadian Centres of Excellence – CCRM and the Institute for Research in Immunology and Cancer - Commercialization of Research (IRICoR) – to launch ExCellThera.

A longstanding goal of Dr. Sauvageau's stem cell research has been to determine the molecular pathways that regulate the activity of HSCs. He made a ground-breaking discovery in the novel small molecule UM171, which causes HSCs to expand in greater numbers while still allowing for rapid neutrophil/platelet recovery in the clinic. This discovery was the result of a collaboration with Dr. Anne Marinier, lead chemist at the Institute for Research in Immunology and Cancer (IRIC) at the University of Montreal.

Dr. Sauvageau and Marinier's molecule, combined with a unique bioreactor technology developed by Dr. Peter Zandstra, a professor and bioengineer with the University of Toronto, is the basis of ExCellThera, a clinical-stage biotechnology company that is focused on developing robust and cost-effective ways of growing blood stem cells for therapeutic use in blood cancers and gene therapy.

ECT-001 is ExCellThera's lead product. It combines UM171 and Dr. Zandstra's fed-batch bioreactor technology that targets the expansion of long-term – describing their regenerative capacity – HSCs. ECT-001 expands blood progenitors more than 100-fold and enables the significant expansion of undifferentiated HSCs, which is anticipated to provide the robust long-term reconstitution of the blood forming system from small samples of HSCs.

"[At the launch of ExCellThera], the cooperation of experts in complementary areas, including a chemist, stem cell technologist and bioengineer, was critical," says Dr. Sauvageau. "ExCellThera is a marriage of interdisciplinary science, along with the support of CCRM and ICIRoR. It's rare to see so many different individuals and organizations work together and have the synergy that we observed."

A significant achievement in 2016 was the initiation of Phase I-II clinical trials, examining ECT-001 for the expansion of HSCs in patients who require stem cell transplantation for the treatment of leukemia and other malignant blood disorders, but who lack suitable donors.

"We are already seeing very encouraging clinical benefits," says Dr. Sauvageau. "This research could become a game-changer in the field of hematopoietic stem cell transplantation."

When asked about upcoming milestones for ExCellThera, Dr. Sauvageau explains his vision.

"A key milestone will be the initiation of two Phase III clinical trials, one in Japan and one in the U.S. comparing expanded cord blood transplants with more standard grafts in leukemia and other blood cancers. Once these are completed, and considering what we currently see in the clinic with our technology, we already envision to eventually tackle immune or inflammatory diseases. This would really be remarkable."

Dr. Sauvageau admits that these advances would not be possible without the benefits of the partnership with CCRM and IRICoR.

"In the early days of a biotechnology company, it is often difficult to obtain funding without losing control of the work," Dr. Sauvageau explains. "In a complex cell therapy program, loss of control would compromise the integrity of products because the founders' expertise is needed. It would have been difficult to launch ExCellThera without CCRM and IRICoR because they empowered the founders to maintain control of the research, while providing the financial and operational support that we needed."

INDUSTRY CONSORTIUM PARTNER

THERMO FISHER SCIENTIFIC AND CCRM – A STRONG PARTNERSHIP **THAT SUPPORTS INNOVATION AND PRODUCT DEVELOPMENT**

For Dr. Kurt Vogel, Associate Director, External R&D at Thermo Fisher Scientific, working with an innovative partner who acts as an extension of his team is critical for being on the forefront of product development. As a founding member of CCRM's Industry Consortium, Thermo Fisher Scientific has benefited from its relationship with CCRM.

Advancing the technologies that underlie products used in regenerative medicine research is a key component of Thermo Fisher Scientific's success. As a member of CCRM's Industry Consortium, Thermo Fisher Scientific and Dr. Vogel have visibility to new life sciences technologies developed at academic institutions in CCRM's network. However, there are many steps involved to successfully bridge between an academic discovery and a commercial product.

"There's really a much bigger gap than most people recognize [between academic discoveries and commercially-ready products]," Dr. Vogel explains. "Many of the technologies that Thermo Fisher Scientific commercializes originate as academic discoveries. What we excel at is making these technologies more robust and simpler to use. But first, an important part of product development is validating these technologies."

To address this challenge, CCRM assists Thermo Fisher Scientific by testing promising discoveries and generating data points that validate their efficacy. From Dr. Vogel's perspective, having different labs and different sets of hands working with products in development, in an advanced stem cell work flow, is a best practice.

Over the years, CCRM has tested and validated different novel technologies for Thermo Fisher Scientific. Most recently, Dr. Vogel enlisted CCRM to assist in the development of a new media system for induced pluripotent stem cells (iPSCs) in the CRISPR/Cas9 gene editing work flow. This new media, StemFlex™, was recently launched to Thermo Fisher Scientific's customers.

"CCRM gives us truly deeper insight into the use of our products in our customers' hands," says Dr. Vogel.

Thermo Fisher Scientific and CCRM have fostered a close partnership with benefits that extend beyond simply delivering on a work plan. "It's difficult science. We're working with the CCRM team as colleagues and getting their feedback, their insight and their experience."

As such, Thermo Fisher Scientific's partnership collaboration with CCRM aligns with its company mission. "Our mission is to enable our customers to make the world healthier, cleaner and safer," says Dr. Vogel. "Stem cell-based technologies have a huge potential to contribute to the understanding of disease and to develop therapies for disease. Our goal is to enable our customers to advance their research into these areas, and to do so faster and better by using our products. By playing a role in developing products that are more robust and user-friendly, CCRM helps us further our mission."

As the regenerative medicine industry advances and grows, the importance of this line of business will continue to expand for Thermo Fisher Scientific.

Thermo Fisher Scientific plans to keep working with CCRM to support its efforts in launching new products, which will enable its customers to conduct increasingly complex and innovative research in the regenerative medicine field.

2
TECHNOLOGIES ADVANCED TOGETHER

12
MONTHS WORKING TOGETHER

FEATURED STAFF

SCALING-UP **OUR EXPERTISE**

Photo, L-R: Patrick Bedford, Steven Keizer, Lise Munsie, Juliana Jy

A REGULATORY EXPERT

Patrick Bedford

Master of Bioethics and Health Law (MBHL), Regulatory Affairs Certification (RAC) – Manager, Clinical Translation & Regulatory Affairs (Build Unit)

Patrick is a fundamental addition to CCRM's business and consulting services team. With 10 years of experience at Health Canada interpreting federal regulatory requirements for emerging biotech products, including cell and gene therapies, Patrick offers a wealth of knowledge to CCRM and our partners. Patrick can help you overcome regulatory barriers, interact with regulators, and progress towards the clinic.

A STRATEGIC THINKER

Juliana Jy

BSc (Honours), Dip. Pharmaceutical Regulatory Affairs – Operations & Compliance Manager (BridGE Unit)

Having joined CCRM shortly after the launch of BridGE, Juliana has played a strategic role in designing the internal structure and operations of CCRM, BridGE and the CATCT facility. Juliana brings quality assurance, supplier quality and operations managerial knowledge to CCRM, where she is revising and transforming standard operating procedures (SOPs), policies and lean manufacturing techniques, ensuring continuous compliance with good manufacturing practice (GMP) regulations and regulatory affairs.

A DEDICATED ACHIEVER

Lise Munsie

PhD – Research & Development Scientist (Advance Unit)

Lise is a vital contributor to CCRM's Advance team. With a PhD in Biochemistry and Biomedical Science from McMaster University and several triathalons under her belt, her dedication and drive do not go unnoticed. Lise spearheads CCRM's induced pluripotent stem cell (iPSC) reprogramming core facility and gene editing program, and leads successful patient and disease specific isogenic iPSC line contract projects.

A QUALIFIED LEADER

Steven Keizer

MBA – Quality Assurance Manager (Deliver Unit)

Steven is leading operations for CCRM's Deliver business unit, which involves designing, developing and implementing systems to support the GMP Contract Manufacturing facility. Steven has extensive GMP consulting and quality assurance management experience, including the design, operations and manufacturing site support of several work sites. Steven has degrees in Chemistry from Western University and an MBA from the Sauder School of Business.

COMPANY CREATIONS

CCRM'S ROLE CRITICAL IN THE LAUNCH OF A **SIGNIFICANT STEM CELL INVESTMENT IN CANADA**

Between a new stem cell therapeutics company being launched in Toronto and the news that CCRM's portfolio companies – AVROBIO, Kisoji Biotechnology Inc. (formerly Actium Research) and ExCellThera – all secured financing to support their clinical trials, 2016 ends on a start-up cluster high note.

Bayer AG and Versant Ventures, a U.S. venture capital firm behind several recent investments in regenerative medicine in Canada, have invested $225 million to establish BlueRock Therapeutics in Toronto. BlueRock will focus on developing stem cell therapies for cardiovascular diseases and treatments for Parkinson's. This significant investment is purported to be one of the largest ever initial venture capital financing deals for a biotech start-up.

BlueRock Therapeutics will focus on human pluripotent stem cell (PSC)-derived cell therapies. The company is founded on the intellectual property (IP) of Drs. Gordon Keller (University Health Network and McEwen Centre for Regenerative Medicine) and Michael LaFlamme (Toronto General Research Institute, UHN) and protocols and IP developed by CCRM.

CCRM has been conducting cardiomyocyte manufacturing for the last three years and working on a PSC expansion project since its launch. CCRM is collaborating with BlueRock Therapeutics to share IP, know-how and expertise on induced PSC expansion and differentiation in stirred-tank bioreactors. CCRM's Advance team has successfully generated >3 billion cells per bioreactor, a capabilitiy that enables Dr. LaFlamme's large animal pre-clinical studies. Dr. LaFlamme's work is funded by both Medicine by Design (in partnership with the Ted Rogers Centre for Heart Research) and the Ontario Institute for Regenerative Medicine.

BlueRock expects to hire 50 employees and establish a manufacturing division within the MaRS Centre in downtown Toronto. CCRM will support BlueRock through its establishment and growth and will participate in co-development projects. BlueRock will have access to CCRM's GMP facility and have the option to conduct process development and contract research with the BridGE team in the future.

VIPs at BlueRock Therapeutics announcement, Dec. 12, 2016

Premier Kathleen Wynne welcomes BlueRock to Toronto

Photos courtesy of: Queen's Printer for Ontario, 2016

THE ONTARIO INSTITUTE FOR REGENERATIVE MEDICINE

ADVANCING REGENERATIVE **MEDICINE RESEARCH AND INNOVATION**

The Ontario Institute for Regenerative Medicine (OIRM) was launched with a vision to revolutionize the treatment of degenerative diseases, making Ontario a global leader in the development of stem cell-based products and therapies. OIRM engages more than 200 researchers and 44 partnerships representing industry, universities, hospitals and not-for-profits.

OIRM is CCRM's partner in advancing regenerative medicine research and innovation. OIRM and CCRM work together to create a robust pipeline through which stem cell research discoveries and early stage clinical and commercial applications can become new therapies, new products and generate new jobs in Ontario with medical and economic benefits for Canada and for patients around the globe.

For example, CCRM has reviewed a number of disclosures from OIRM investigators this year and progressed three technologies into due diligence. Of these, provisional patents have been filed on two technologies that have been optioned to CCRM for commercialization and likely to be out-licensed.

"Regenerative medicine is key to driving progress and growth in Ontario's innovative life sciences sector. We are pleased to support the work of OIRM and CCRM. Their partnership brings together basic research, clinical studies and commercialization, which will lead the translation of stem cell and regenerative medicine research into improved health outcomes and economic growth in Ontario," says Reza Moridi, Ontario's Minister of Research, Innovation and Science.

In June, OIRM welcomed Dr. Duncan Stewart as its new President and Scientific Director, replacing founding President Dr. Janet Rossant. Dr. Stewart is a pioneering Canadian cardiovascular researcher, recognized for his many important discoveries in blood vessel biology, as well as his skill in translating these discoveries into cell therapy clinical trials for the benefit of patients and society. He joined OIRM with leadership experience from positions at St. Michael's Hospital and the University of Toronto, and has ongoing roles at The Ottawa Hospital and the University of Ottawa.

OIRM has made significant progress in realizing its five-year mandate through an investment from the Ontario Ministry of Research, Innovation and Science.

19 research projects currently funded, including 4 Disease Team, 3 Accelerator and 12 New Ideas grants

OIRM PIs are leading 8 cell therapy clinical trials ranging from neural repair in children to cell therapy treatments for septic shock

Host organization for the final workshop of the Ontario-China Stem Cell Research & Commercialization Partnership Program, a 3-year collaborative research initiative between Ontario and China that includes CCRM

Developed partnerships with 9 health charities with commitments for co-sponsorship of Postdoctoral Fellowship Awards. Hosted forums and focus groups with health charities and their patient stakeholder groups to increase collaboration and align patient engagement initiatives

33 Ontario-based technologies assessed, 13 patent applications, 9 provisional patents, 4 new licenses and 8 active licenses

Hosted 3 workshops through OIRM's Clinical Trials Initiative. Topics included introduction to clinical trials, Health Canada regulations, and economic reimbursement modelling

MEDICINE BY DESIGN

INNOVATIVE COLLABORATION **DRIVES DISCOVERIES TO MARKET SOONER**

When more than 90 researchers at a top-ranked global research university converge around key problems in regenerative medicine, breakthroughs are bound to emerge. But what happens next to translate those discoveries into new therapies and products that benefit patients and drive economic growth is just as critical.

That's the thinking behind CCRM's partnership with Medicine by Design. Medicine by Design harnesses the expertise at the University of Toronto and its affiliated hospitals at the convergence of physical and life sciences, engineering, mathematics and medicine to undertake transformative research in regenerative medicine and cell therapy. Through multidisciplinary collaborations, the use of engineering design principles and quantitative biological modelling and strategic recruitment, Medicine by Design is building a robust engine of discovery that will drive a new innovation hub for regenerative medicine in Toronto.

CCRM is a crucial partner in this process. By assessing the clinical translation and commercialization potential of Medicine by Design's 20 team projects and connecting researchers to industry at an early stage, CCRM is helping Medicine by Design to accelerate discoveries from the laboratory bench to the market, benefiting patients sooner. Medicine by Design's research, combined with CCRM's deep connections with industry and regulatory expertise, are creating a seamless pipeline that will establish Canada as a leader in the development and manufacture of cell-based therapies. This productive collaboration is poised to deepen in 2017 when Medicine by Design and CCRM staff move into new space in the MaRS Discovery District, in close proximity to the University of Toronto and several of its affiliated hospitals, creating a unique centre of excellence in regenerative medicine.

Medicine by Design is made possible by a grant from the Canada First Research Excellence Fund.

UNIVERSITY OF TORONTO

MEDICINE by DESIGN

CANADA FIRST RESEARCH EXCELLENCE FUND | APOGÉE CANADA FONDS D'EXCELLENCE EN RECHERCHE

TRAINING & EDUCATION

A COMMITMENT TO **ADVANCING KNOWLEDGE**

Every year, CCRM hosts and participates in several events to promote knowledge translation and add to the discussion around commercializing regenerative medicine, and cell and gene therapies. Giving highly qualified personnel opportunities to advance their knowledge is a responsibility we take seriously, along with others in the community.

CCRM's Commercialization of Tissue Engineering Workshop – 10th World Biomaterials Congress: May 17, Montreal, QC

The World Biomaterials Congress is one of the largest gatherings of biomaterials scientists, with over 3,500 abstracts submitted from more than 60 countries. CCRM's pre-conference workshop provided a detailed understanding of best practices, regulation and successfully applied strategies for translating academic discoveries into marketable products, from industry entrepreneurs and business leaders.

The Business of Regenerative Medicine – How to Build a Company: July 11-13, Boston, MA

The Business of Regenerative Medicine course held at the Harvard Business School focused on critical issues associated with conceptualizing, developing and building a regenerative medicine company. This course is the 9th in a series organized by Case Western Reserve University, CCRM, Harvard Stem Cell Institute and the Parker H. Petit Institute for Bioengineering & Bioscience at Georgia Tech. In 2017, CCRM will be hosting the course in Toronto.

2016 Till & McCulloch Meetings: October 24-26, Whistler, BC

The Till & McCulloch Meetings (TMM) are Canada's premiere stem cell research event, co-hosted by CCRM, Stem Cell Network and the Ontario Institute for Regenerative Medicine. TMM are an annual homecoming for over 400 leading stem cell scientists, clinicians, bioengineers and ethicists as well as representatives from government and industry in Canada and around the world. CCRM hosts TMM as part of our mission to facilitate global collaboration and support the development of foundational technologies in cell and gene therapies and regenerative medicine technologies.

Cell & Gene Therapies Workshop – Regulatory and Manufacturing Issues: October 27, Whistler, BC

CCRM's BridGE and Advance business units came together to deliver a workshop on the regulatory and manufacturing issues and considerations involved with cell and gene therapies, following the 2016 Till & McCulloch Meetings. This workshop featured informative talks and two collaborative problem solving exercises on the processes required to get pre-clinical cell and gene therapies commercially ready.

Science Communications Workshop: November 24, Toronto, ON

Led by the Ontario Institute for Regenerative Medicine, CCRM participated in this workshop as an expert in the field, educating graduate students and early career researchers on how to present research in plain language using media interviews, writing and social media to develop successful communication styles as scientists.

OIRM science communications workshop

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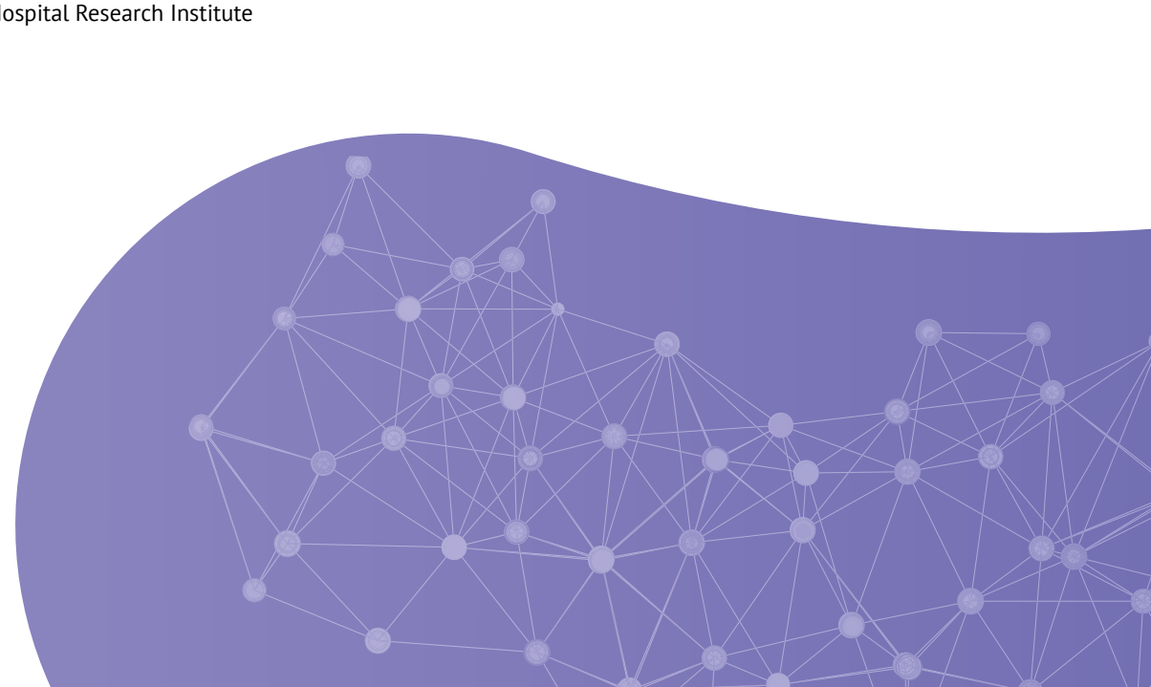

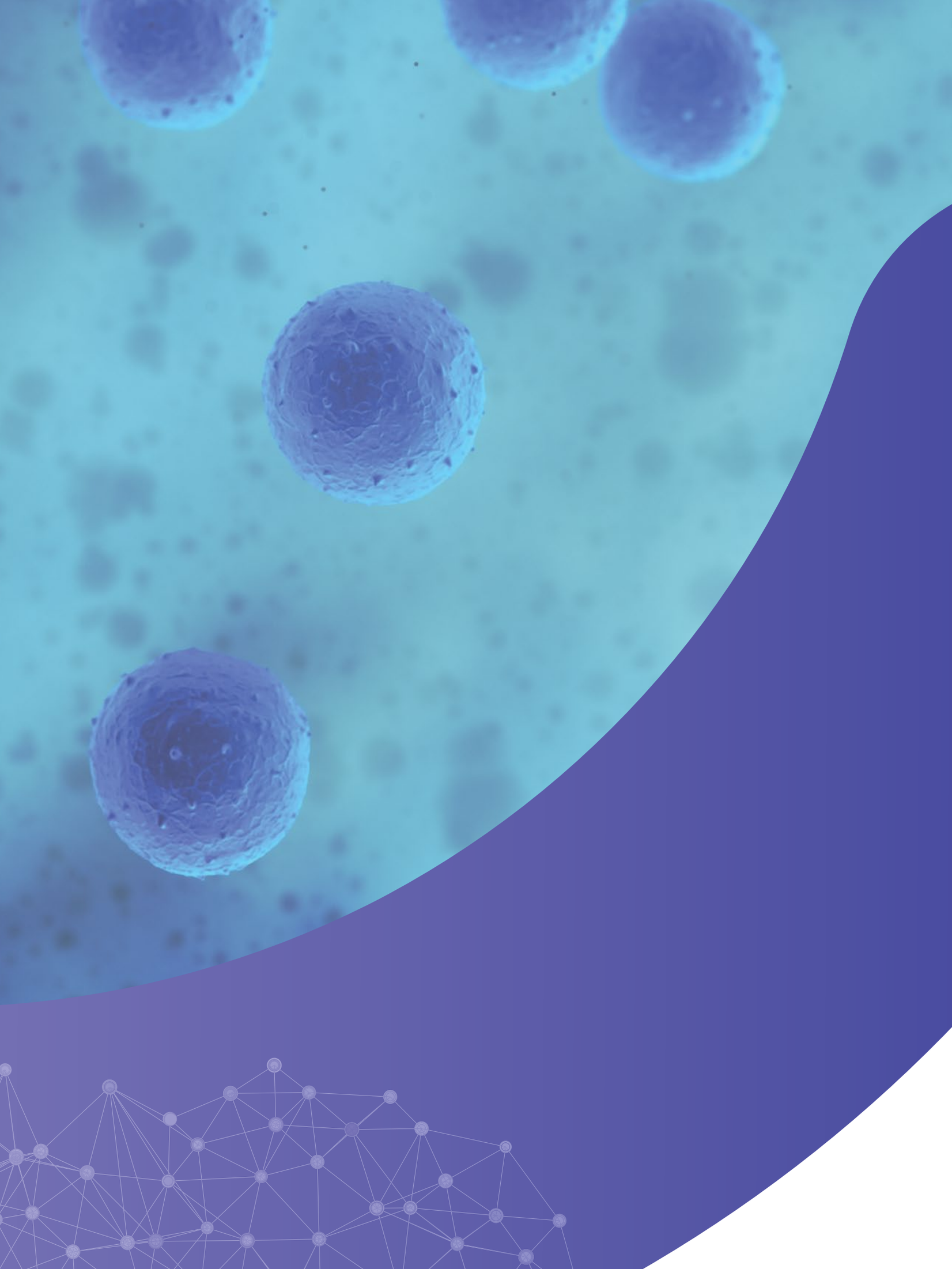

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