

CHALLENGE ACCEPTED

2020 ANNUAL REPORT



Commercializing
Living Therapies



CCRM is a Canadian, public-private partnership supporting the commercialization of cell and gene therapies with strategic funding, dedicated infrastructure and specialized business and scientific expertise. By partnering with leading research institutions to launch new ventures, enabling industry by providing innovative contract development and manufacturing organization (CDMO) services, and scaling emerging companies by catalyzing investment, CCRM is accelerating the translation of promising technologies, processes and therapies into life-changing health outcomes for patients.

OUR PURPOSE

CCRM is revolutionizing health care by solving the big problems in regenerative medicine.

OUR VISION

To be the preferred partner 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.

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WORKING SAFELY

CCRM's priority is always the health and safety of our staff. In 2020, CCRM remained open to lab and facility employees, with other teams working from home. All COVID-19 safety requirements were followed to keep our people and essential visitors safe when on-site.

All of the photography included in this year's annual report was either taken before March 2020 (when COVID-19 changed work and life in Canada), or physical distancing practices were in place and those not being photographed wore masks.



BOARD OF DIRECTORS

Michael Nobrega (Chair)
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Reni Benjamin
JMP Securities LLC

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Cytiva

Vivek Goel
(January to June)
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Sven Kili
Antion Biosciences

Michael May
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Gail Naughton
Histogen, Inc.

Christopher Paige
University Health Network

Edward (Ted) Sargent
(July to December)
University of Toronto

Joan Sheehan
STEMCELL Technologies

Phil Vanek
Gamma Biosciences

Donna Wall
The Hospital for Sick Children

Peter Zandstra
University of British Columbia

Claudia Zylberberg
Akron Biotech

CELEBRATING THE POSITIVE IN 2020 AND BEYOND

When we look back on 2020, I expect several themes will emerge including innovation, perseverance, resilience and collaboration. Also of note, it was a good year for science, a great year for biotech, and an excellent year for the global cell and gene therapy (CGT) industry that saw record investments of US\$15.9 billion in the first three quarters of 2020 ([Alliance for Regenerative Medicine](#)).

Facing a global pandemic, pharmaceutical and biotech companies rallied around the challenge of finding a vaccine and, in record time, they did. In less than one year, Pfizer and BioNTech, AstraZeneca and University of Oxford, and Moderna all produced viable products. What typically takes many years happened in less than 12 months and, especially worth noting, regulatory approvals happened just as quickly. Science saved the day and public health officials became household names.

Around the world we saw companies and individuals reacting to the new reality by adjusting, adapting and getting innovative. Commerce and conferences moved to online and virtual platforms, and workforces were able to stay connected once they mastered new technology. While “Zoom fatigue” became part of our lexicon, virtual tools were also our saviour. It allowed CCRM and other companies to remain productive,

and we realized that COVID-19 had kick-started a paradigm shift that likely would have happened anyway, but the timelines were accelerated.

In the face of adversity, I’m proud of how the team at CCRM reacted. We kept our personnel safe and healthy, continued to meet (and exceed!) our clients’ expectations, supported the community through funding and participation at virtual events, and did our utmost to maintain a caring, collegial and connected culture within and among our business units, despite having half of our employees working remotely. No easy feat.

In case you missed the news in November, CCRM and McMaster Innovation Park signed a letter of intent to build and operate a 120,000 sq. ft. facility to produce cells and viral vectors for

Phase III and commercial-scale manufacturing for CGTs. It will be the largest facility of its kind in Canada and we're extremely pleased that this strategic goal is coming to fruition.

We also made progress on other key fronts: thanks to our renewed partnership with Cytiva, our scientists and engineers will continue to tackle cost of goods in manufacturing by developing, optimizing and integrating processes and tools. Our Good Manufacturing Practices (GMP) facility, a partnership with University Health Network, was able to maintain operations and serve industrial and academic clients. And we continued to grow our workforce.

The CCRM team established an initiative in 2020 to formalize some of our ad-hoc events around professional development, equity, diversity and inclusion (EDI), and social/cultural activities. The Equality, Professionalism and Inclusiveness Council (E.P.I.C.), with the goals of celebrating diversity at CCRM, helping those in underrepresented groups to grow professionally, and empowering employees to make a contribution at work and in their communities, was a welcome addition to the culture at CCRM, especially in a year where we couldn't all be together. A heartfelt thank you to the E.P.I.C. volunteers and to the employees who embraced the new programming.

With 2020 behind us, we are excited about 2021 – our 10 year anniversary! Over the next 12 months, join us as we celebrate a new theme each month that recognizes our milestones, partnerships, people and more. We hope you enjoy reminiscing with us on our social media platforms as we roll out our #CCRM10Years campaign. This first decade has been a blast!



Michael May
President and CEO



Photo taken in December 2019

YOUR VISION. OUR PURPOSE. HEALTHIER LIVES.

A great deal of time and effort has gone into establishing CCRM as a contract development and manufacturing organization (CDMO) to serve the needs of industry and academics producing cell and gene therapies. With the team in place and projects underway in the Centre for Cell and Vector Production (CCVP), and the Centre for Advanced Therapeutic Cell Technologies boasting more than four years of operations, we are ready to help you.

We solve the big challenges in cell and gene therapy through seamless integration of:



EXTENSIVE EXPERTISE

Industry-leading team with 35+ PhDs; significant breadth and depth of specialized expertise



ENABLING TECHNOLOGIES

Access to cutting-edge, end-to-end technologies through industry partnerships



COLLABORATIVE PROCESSES

Collaborative and transparent client-centric approach to problem solving



SPECIALIZED INFRASTRUCTURE

Co-located PD and GMP manufacturing to provide continuity of process from lab to clean room

CCRM'S EXPERTISE IN DEVELOPING, SCALING UP AND MANUFACTURING CELL AND GENE THERAPIES INCLUDES:

- iPSC reprogramming, gene editing, differentiation
- Hematopoietic stem cell (HSC) isolation and processing
- Master/working cell banking
- Automated, closed, integrated T-cell process development (PD) and manufacturing (e.g. CAR-T)
- Scale-out and scale-up for autologous and allogeneic therapies, respectively
- Viral vector (lentivirus, adeno-associated virus, other) up and downstream process development
- Analytical development including high-throughput screening assays, quality control (QC) testing, and ICH stability studies
- In-house QC lab to support product release
- Custom and high-throughput media development
- Cryopreservation strategies

CCVP TEAM
IN 2017



CCVP TEAM
IN 2020



Digital composition. Subjects were photographed individually following COVID-19 protocols.

2020 AT A GLANCE

The year started well, but then work slowed as the world attempted to navigate productivity in a pandemic. But people are resilient, and as the year wrapped up it was apparent that deals happened, innovation saved the day and CCRM has many shining stars in its constellation.



Simon Ellison: A leader in his field – January

CCRM and CellCAN kicked off 2020 with a free event in Toronto that featured international experts and health care leaders discussing their experiences and solutions to supply chain and logistics challenges. Naturally we invited Simon Ellison, of World Courier, for his deep knowledge and insight. It was a pleasure to work with him and we are still saddened by his passing.

INNOVATION ECONOMY COUNCIL

Data-driven insights for Canadian innovation – April

In response to the COVID-19 crisis, MaRS Discovery District invited a group of industry leaders, like CCRM and others that advocate on behalf of startups, to form a council to explore how Canada's established companies and new technology ventures can help shape a new industrial innovation policy for the country. Learn more about the [Innovation Economy Council](#).



Cytiva renews funding for CATCT – June

The successful partnership between CCRM and Cytiva was given the green light to proceed for another three years. The Centre for Advanced Therapeutic Cell Technologies (CATCT) was launched in 2016 to accelerate the development and adoption of manufacturing technologies for novel cell and gene therapies. CATCT addresses the manufacturing bottlenecks that would otherwise have the potential to impede the industry's growth.

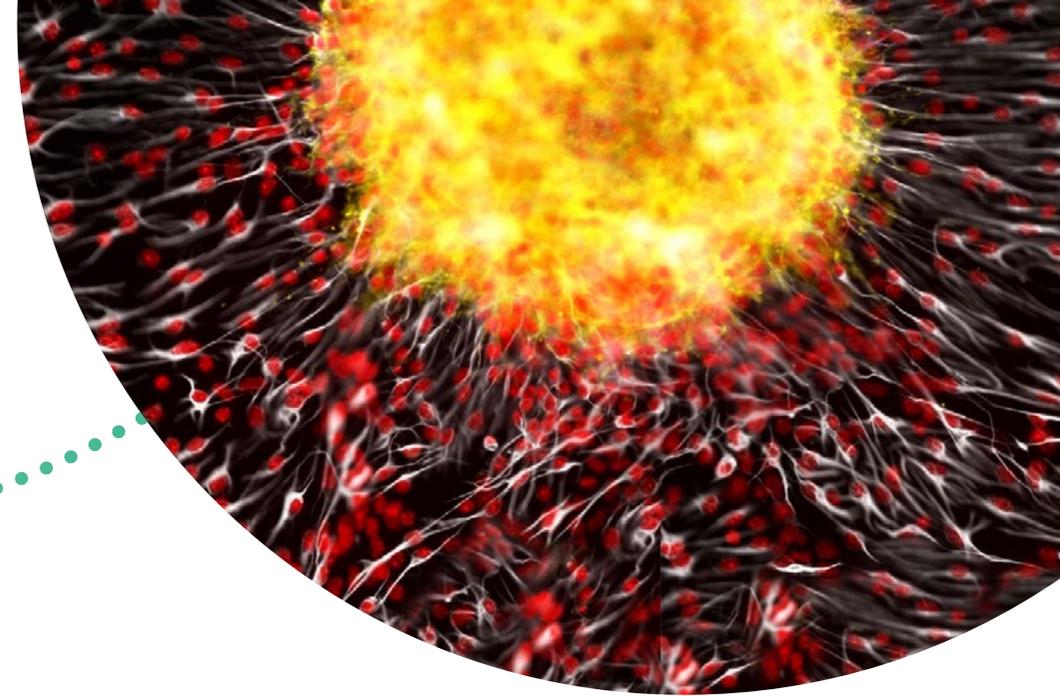


A fresh take on our website – September

CCRM's website had a brand new look when we unveiled our new site in September. Working with Public Good Social Marketing Communications once again, we introduced a playful and interactive design and showcased our key pillars: fee-for-service, company creation and investing. Have a [look](#) and make sure to play with the "cells."

CELLS I SEE

Congratulations to Danielle Spice, of Western University, winner of the 2020 Cells I See Grand Prize for Cell Supernova. The Grand Prize winner is selected based on blind voting by delegates at the annual Till & McCulloch Meetings.



CCRM to operate Canada's largest CDMO – November

CCRM and McMaster Innovation Park (MIP) signed a [letter of intent](#) to develop and operate a biomanufacturing campus in Hamilton, Ontario. The parties will support the construction of a contract development and manufacturing organization (CDMO) to produce cells and viral vectors for Phase III and commercial-scale manufacturing of cell and gene therapies. This will anchor MIP as the western end of southern Ontario's Life Sciences Corridor.

Congratulations to our friends and advisors for these significant achievements and awards in 2020!



Peter Zandstra,
inducted into the
Canadian Academy of
Health Sciences



Molly Shoichet,
winner of the Gerhard
Herzberg Canada Gold
Medal for Science and
Engineering, and Top 100
Most Powerful Women
in Canada



Michael Rudnicki,
Fellow of the Royal
Society (UK)



Gordon Keller,
winner of the Bloom
Burton Award



**Kim Warren and
Azadeh Golipour**
(AVROBIO), winner
National Xconomy Awards,
Secret Weapon Category

HOW BLUEROCK THERAPEUTICS IS ADVANCING NOVEL “OFF-THE-SHELF” THERAPEUTICS

Simply operating as usual in 2020 was a feat unto itself. But BlueRock Therapeutics didn't allow the challenges that arose during a global pandemic to impede progress toward its goal of one day helping patients gain access to novel therapeutics. It continued to advance programs in its therapeutic areas of focus: neurology, cardiology and immunology.

BlueRock Therapeutics, a wholly-owned subsidiary of Bayer AG, moves promising pluripotent stem cell programs forward to possible therapeutic candidates. It was founded in 2016 by Versant Ventures and Bayer AG with the second largest-ever Series A financing for a biotech. With sites in Toronto, Canada, and New York City and Cambridge in the United States, BlueRock has gained footholds in North America's top biotechnology hubs.

BlueRock's novel *cell+gene* platform advances the work of its scientific founders into what may one day be off-the-shelf therapies that address degenerative diseases in new ways. Drs. Gordon Keller and Michael Laflamme, based in Toronto, have expertise in cardiac disease and hematopoietic differentiation, and Drs. Lorenz Studer and Viviane Tabar, based in New York City, have expertise in a specific dopamine neuron for Parkinson's disease.

When reflecting on all the work done so far, Derek Hei, PhD, Chief Manufacturing and Technical Operations Officer at BlueRock, shared what makes the company's proprietary *cell+gene* platform unique.

“We look at diseases that really can’t typically be addressed by gene therapies, in that the cells that are there are either very sick or dead and need to be replaced. Our niche focuses on the potential of replacement with authentic cells, and then adding a layer of gene editing to help enhance the therapeutic activity of those cells. Creating off-the-shelf, allogeneic [from a donor] cell therapy products is our goal.”

Work is underway to get the first therapeutic candidate for the treatment of Parkinson’s disease into North American clinical trials. BlueRock is also advancing its other therapeutic candidates in the pipeline, which are in the cardiac and immunology spaces.

Dr. Hei shared that engagement with the CCRM team has helped to enable the accomplishments to-date, and will likely benefit BlueRock into the future.

“CCRM has really been a partner for us in holding up the GMP part of the platform. We interacted with teams from CCRM in the very beginning to support the banking of unedited pluripotent stem cells. Now, the advantage of having CCRM as a partner is that it gives us the flexibility that we need to address the growing pipeline. We have our own internal manufacturing capabilities, but there’s no way our capabilities will be able to address all of our needs down the road.”

Dr. Hei is mindful that BlueRock’s novel treatments may one day make significant impacts in the lives of people living with conditions in their areas of focus.

“We are very patient-focused, and we’re hopeful that we will be helping patients living with Parkinson’s disease in a very significant way down the road. We’re hoping that we’ll have similar products in the cardiac and immunology spaces following along not too far behind that.”



Derek Hei, BlueRock Therapeutics

NEW ACADEMIC ADVISORS ADVANCE EFFORTS TO COMMERCIALIZE EMERGING TECHNOLOGIES

To help extend CCRM's efforts in the identification and commercialization of new regenerative medicine technologies, and cell and gene therapies, we established an advisory committee comprised of leading Canadian academics and experts, in July 2020.

Working closely with CCRM's team responsible for identifying and developing promising technologies and nurturing them to the company creation stage, the committee was struck to advise, inform and connect us with promising new research.

The committee members have already introduced us to a number of new contacts and inventions, and we look forward to building on this foundation in the future. Their names and photos appear on the page that follows.

CCRM acknowledges the support of the participating academics and experts, representing the breadth and depth of knowledge that exists nationwide within the regenerative medicine and cell and gene therapy field.



CORINNE HOESLI, PhD

Associate Professor
McGill University
Montréal, Québec



NIKA SHAKIBA, PhD

Assistant Professor, School of Biomedical
Engineering, University of British Columbia
Vancouver, British Columbia



DAVID JUNCKER, PhD

Professor and Chair of Biomedical
Engineering Department at McGill University
Montréal, Québec



STEPHANIE WILLERTH, PhD

Full Professor and Canada Research Chair
in Biomedical Engineering,
University of Victoria
Victoria, British Columbia



MICHAEL KALLOS, PhD

Professor, Schulich School of Engineering
and Director Biomedical Engineering Calgary
Initiative, University of Calgary
Calgary, Alberta



ALLISON MCGUIGAN, PhD

Professor, Department of Chemical Engineering
and Applied Chemistry and Institute for
Biomedical Engineering, University of Toronto
Toronto, Ontario



NICHOLAS PINEAULT, PhD

Sr. Scientist and Manager
Canadian Blood Services
Ottawa, Ontario

FEATURED STAFF

COMMITTED TO OVERCOMING CHALLENGES IN INNOVATIVE AND COLLABORATIVE WAYS



ATEFEH GHORBANI, PHD
Development Manager

Atefeh began her career identifying cell-culture processes to enhance therapeutic antibody production and transitioned to the field of regenerative medicine. She joined CCRM as a Development Scientist bringing years of industry experience in biopharmaceutical engineering and bioprocess manufacturing. Managing the process development team on the large-scale production of gene therapy products, she provides leadership in technical expertise, strategy and planning, as well as a passion for mentoring her colleagues.



CYNTHIA LAVOIE, PHD, MBA
President and Chief Investment Officer
CCRM Enterprises

Cynthia is a seasoned investment professional with 18 years in venture capital and an accomplished academic with a strong scientific background in R&D. Her expertise includes creating companies *de novo* and leading investments into companies developing therapeutic drugs and technologies. Cynthia is at the helm of CCRM Enterprises Ltd., the for-profit venture investment arm of CCRM, which invests in early-stage companies developing therapeutics in cell and gene therapy.

79% of employees have graduate-level degrees

86% come from STEM disciplines

CCRM employees speak 39 different languages



VISHAN SIVAGNANAM, MSC, MBA
Commercialization Manager

Vishan leads CCRM's strategic support activities for Medicine by Design, a regenerative medicine initiative at the University of Toronto. He brings over a decade of experience in commercializing early-stage discoveries. In his role, he provides tailored commercialization advice to academic investigators including strategic recommendations to inform decisions around translational planning and commercial positioning. Vishan also supports CCRM's company creation activities.



ROBIN QUIRK, PHD, MBA
Director of Technology Sourcing and Venture Development

Robin has over 20 years of experience working with regenerative medicine start-up companies. He started his career as an industry-trained pharmacist before moving into tissue engineering research. Robin joined CCRM to support the commercial development of early-stage ideas. He leads the team responsible for identifying and developing promising technologies and nurturing them to the company creation stage by strategically leveraging CCRM's expertise and infrastructure.

INSPIRING MINDS WITH VIRTUAL TRAINING AND EDUCATION

As virtual conferences and workshops became the norm for offering training and educational opportunities as of March, we were pleased to have already hosted an in-person seminar by then. Here's how CCRM engaged trainees and others in the regenerative medicine, and cell and gene therapy fields, with educational content this year.

In January, CCRM partnered with CellCAN to host the first **Supply Chain and Logistics for Cell and Gene Therapies Seminar**. It took place in Toronto and, as a harbinger of things to come, a virtual webcast was also offered.

International experts and health-care leaders discussed their experiences and solutions to supply chain and logistics challenges for the industry, and shared key considerations for the first and last 100 metres to get a treatment to the clinic. Presenters touched upon global issues, the regulatory environment, how product is received on the clinic floor, and the physician's experience. You can watch the five presentations on [our website](#).

Building upon the success of the North American **Business of Regenerative Medicine** event (read about the 2019 edition [here](#)), an inaugural Asia Pacific version was planned by co-hosts CCRM Australia and the NSW Stem Cell Network for April. The in-person symposium was postponed and the co-hosts [offered a free webinar](#) in its place.

Experts from Australia, China and Canada discussed how the regenerative medicine sector was coping with the effects of the pandemic, expected impacts on the future, and strategies and practical suggestions for working post-pandemic.

In recent years, the University of Toronto's Medicine by Design has hosted **Summer by Design** with CCRM and the Rotman School of Management. Adapting this opportunity to the challenges of 2020 meant a pivot to a new webinar series, called **Translation Talks**. See page 17 for a description.

The **Till & McCulloch Meetings (TMM)**, Canada's premier stem cell research event, took place from October 26-28. TMM 2020 brought together over 400 stem cell scientists, clinicians, ethicists, industry experts and policy-makers from Canada and abroad virtually for the first time. While the quality of the conference and the speakers was exceptionally high, we look forward to being all together in Ottawa in 2021. CCRM took a break from co-hosting duties and instead supported TMM as the Platinum sponsor.

Lastly, CCRM was the first stop on a **virtual tour of Canada's cell and gene therapy manufacturing facilities**, hosted by CellCAN. On December 17, a webinar in this series highlighted the Centre for Cell and Vector Production, the GMP facility that CCRM operates in partnership with University Health Network. We educated participants about the facility and the important work being done on-site.



PARTNERING TO OVERCOME CHALLENGES, EVEN GRAND ONES

Medicine by Design is a regenerative medicine initiative at the University of Toronto that undertakes transformational research to improve health outcomes and create value. CCRM has been its commercialization partner since it launched in 2015.

As a result of the COVID-19 pandemic, Medicine by Design and CCRM were unable to host the fourth edition of “Summer by Design.” Instead, with the Stem Cell Network, we offered “Translation Talks,” a seven-week webinar series that introduced participants to key concepts required for the translation and commercialization of regenerative medicine discoveries, including market diligence, intellectual property, regulatory affairs, early-stage investment and manufacturing. Summer by Design welcomes international PhD candidates and post-doctoral fellows to Toronto for an intensive academic experience. The online Translation Talks was able to accommodate a larger pool of participants, from graduate students through to research/scientific associates – the silver lining.

During the year, CCRM continued to support Medicine by Design’s newly-funded Cycle II projects by offering strategic recommendations around translational planning and commercial positioning.

Medicine by Design also launched a new Grand Questions program in July centred around developing transformative and revolutionary solutions that will be of critical importance to regenerative medicine over the next 20 years. CCRM is looking forward to supporting future discoveries that emerge from this ambitious program.



Participants and organizers at Summer by Design 2019 in Toronto

KEY FUNDERS



Federal Economic Development
Agency for Southern Ontario
Agence fédérale de développement
économique pour le Sud de l'Ontario



FOUNDING INSTITUTIONAL MEMBERS



ASSOCIATE INSTITUTIONS



CURRENT AND FORMER* PORTFOLIO COMPANIES



COLLABORATIVE PARTNERS



INDUSTRY CONSORTIUM

CCRM has established a consortium of more than 100 companies that represent key sectors of the regenerative medicine industry, including therapeutics, devices, reagents, and cells as tools. These companies range from multinational corporations, to small-medium enterprises, to emerging start-ups. They have been able to utilize the translational platforms developed by CCRM to enable new opportunities and address real-life bottlenecks in their businesses.

CCRM would like to acknowledge the valuable relationships that have been fostered with these companies. We believe all parties have benefited from the synergies created through the industry consortium.

CCRM

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