

THE FUTURE IS UNFOLDING

FROM CELLS TO SOLUTIONS

2017 ANNUAL REPORT



Commercializing
Living Therapies

CCRM is a Canadian, not-for-profit, public-private consortium supporting the development of foundational technologies that accelerate the commercialization of regenerative medicines, including cell and gene therapies. We bridge the regenerative medicine commercialization gap by leveraging funding and infrastructure, and mobilizing business and scientific expertise to translate technologies into revolutionary products that are accessible to patients.

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. Our rich pipeline of new and potentially life-changing cell, gene and regenerative medicine-based treatments will reach patients more effectively if the translation from laboratory to market is accelerated and de-risked in a coordinated, collaborative and capital-efficient manner.

CCRM has assembled a world-renowned network of stem cell scientists, bioengineers, industry partners, investors and entrepreneurs to fulfill the promise of regenerative medicine.

CCRM



OUR MISSION

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.

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People



80+ employees,
plus a network
of industry and
academic partners

Funding



Over \$90 million

Facilities



40,000 ft²
(~3,700 m²) dedicated to
advanced manufacturing and
product development

EXECUTIVE MESSAGE

WE'VE BEEN KEEPING THIS SECRET FOR MONTHS NOW AND WE'RE DELIGHTED TO FINALLY SHARE IT HERE.

The exciting news is that CCRM has received another \$15 million in the latest competition from the Government of Canada's Networks of Centres of Excellence program.

This grant gives CCRM the runway and resources to enhance company creation, establish unique regenerative medicine-focused investment vehicles, strengthen international partnerships, and more. It's endorsement that we are on the right trajectory for building a global regenerative medicine ecosystem from a strong base of leadership in Canada.

Moving into a custom-designed space in the MaRS Discovery District, in early 2017, was a turning point for CCRM. In addition to the advantages of our new location, our staff is finally all together in one space, working in a state-of-the-art advanced manufacturing facility that has enabled collaborations and synergies not previously available to CCRM.

Our scientists and engineers have leading-edge equipment to facilitate their work, develop their programs and support academic and industry clients. The Advance team has attracted significant projects (see the Affigen profile on page 13) and the BridGE team's progress on closed, automated and turn-key manufacturing workflows is driving the industrialization of cell therapy.

Our Deliver team, which you'll read about on page 9, will manage the Centre for Cellular and Vector Production (CCVP) in our space at MaRS. The good manufacturing practices (GMP) facility will manufacture clinical-grade cell and gene therapies for Phase 1 and 2 trials. This will create a seamless link between the process development and scale-up team (BridGE) and state-of-the-art manufacturing and commercialization support.

Finally, our business and regulatory consulting practices are growing, and you can read about CCRM's efforts to launch new companies on page 16. Consistent with our original vision, we continue to work with key partners to help academics and industry bridge the many gaps to commercialization.

2017 was also a pivotal year for regenerative medicine, and the cell and gene therapy sector.

With an estimated 822 regenerative medicine and advanced therapy companies at work in the space and 900 clinical trials underway at mid-year, plus US\$4.25 billion raised in the same timeframe¹, the sector is hot and delivering results.

Importantly, the U.S. Food and Drug Administration granted two landmark approvals for cell-based therapies for cancer and a third for blindness – and two of these therapies achieved reimbursement from the U.S. Centers for Medicare and Medicaid Services. On the investment side, companies in Canada and elsewhere are getting impressive injections of capital to translate ground-breaking science into clinical products. For example, last year we highlighted the work of Dr. Guy Sauvageau (Université de Montreal) and CCRM-IRICoR spin-off ExCellThera with its best-in-class blood stem cell expansion platform. This year, ExCellThera will leverage the completion of its Phase I/II oncology trial into a pipeline of new clinical trials and future products. This represents just one of the many ways that CCRM will *launch, scale and attract* companies within its expanding network.

So, what does all this mean? The sector has reached a tipping point and Canada is ready to ride the wave. With its exceptional employees and many partners, CCRM is building a Canadian regenerative medicine ecosystem that will impact medicine for the next 50 years.



Peter Zandstra
Chief Scientific Officer

Michael May
President and CEO

Greg Bonfiglio
Chair, Board of Directors



THIS IS

Centre for Cell
and Viral
Production
(GMP facility)

CCRM

CCRM marked a significant milestone in 2017

with the move to our 40,000 ft² (~3,700 m²) facility in the MaRS Discovery District. Making the move from multiple sites to one space, with our partners, enables CCRM to collaborate and innovate together in a hub that's truly dedicated to regenerative medicine. If you haven't yet visited us, here's what you would see on your tour.



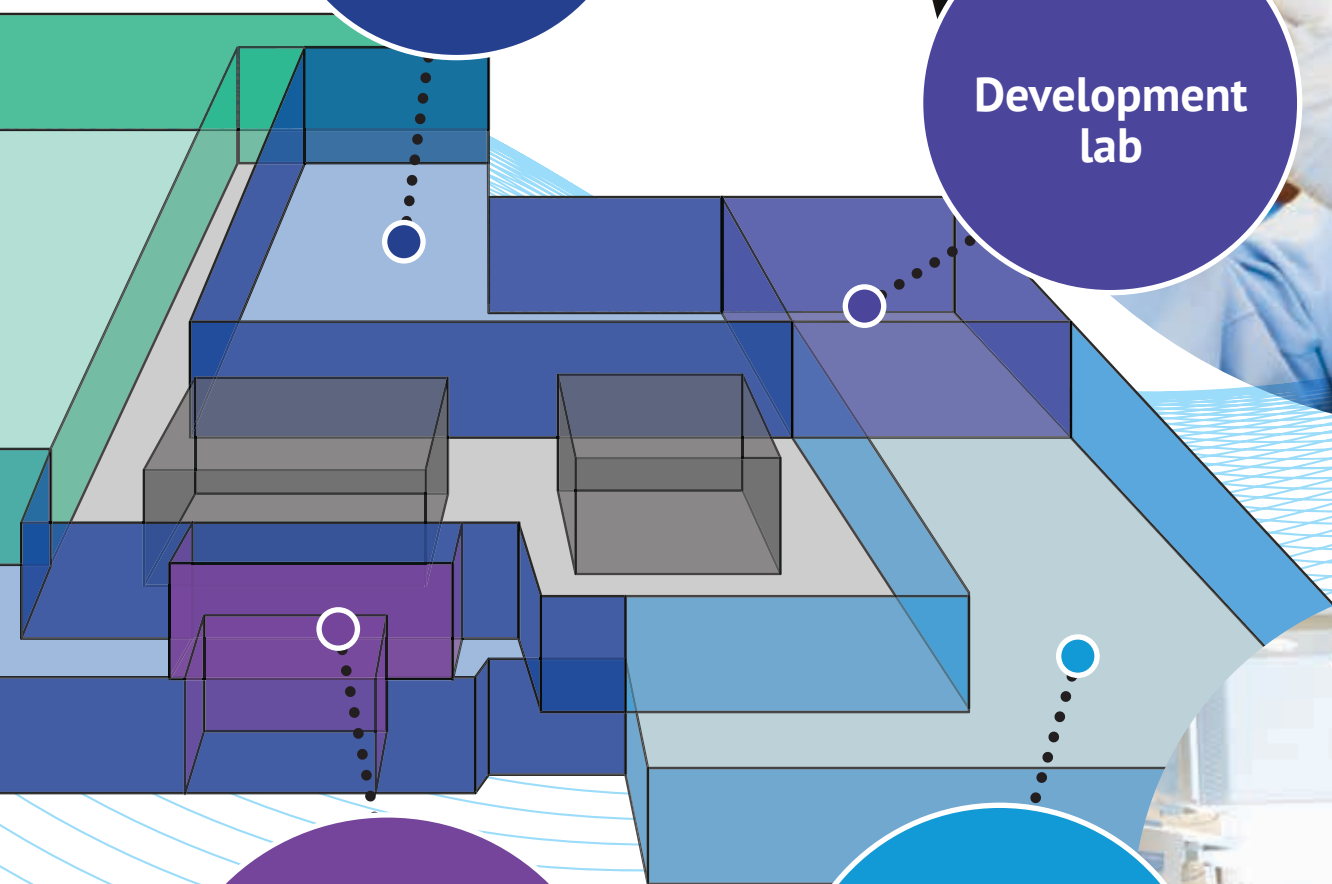
CCRM offices/
workspaces



**CCRM offices/
workspaces**



**Development
lab**



**Medicine by Design
and OIRM offices/
workspaces**

**Centre for
Advanced
Therapeutic Cell
Technologies
(CATCT) lab**



JANUARY TO DECEMBER 2017

A YEAR OF HIGHLIGHTS



MOVING INTO OUR NEW HOME IN MaRS

Having outgrown our existing space and housing staff in three locations, it was a huge treat, in February, to move everyone into a state-of-the-art facility in the MaRS Discovery District in Toronto. Our new office is a showstopper and allows for collaboration and engagement between our technical, business and corporate teams.



THANKS FOR VISITING

While we're still very much in the business of regenerative medicine, the amount of tours we've conducted through our space might indicate otherwise. Between Doors Open Toronto, Toronto Health Innovation Week, foreign governments, foreign media, local governments, local media, our partners, our collaborators, and others, some of us could moonlight as docents.



UNITING TO STRENGTHEN THE CANADIAN RM ECOSYSTEM

CCRM has had the privilege of working closely with its Canadian counterparts this year. From the launch of the Regenerative Medicine Alliance of Canada (RMAC) to the Canadian Pavilion at the International Society for Cellular Therapy (ISCT) conference in London, England, and the Bench to Bedside for Biotherapeutics (B3) training event, we are stronger together.



PORTFOLIO COMPANIES IN THE NEWS

CCRM's first spin-off, ExCellThera, won \$20,000 from global investors at the 2017 MaRS HealthKick Challenge. AVROBIO expanded its clinical pipeline to include gene therapies for Cystinosis, Pompe disease and Gaucher disease. Feldan Therapeutics signed a research collaboration with Amgen to develop and deliver novel intracellular biologics.



INCREASING OUR DIGITAL FOOTPRINT

It's good to try new things, but stick with what works is a good adage too. CCRM did both this year. In October, we launched a live video segment on Facebook to broadcast topics of interest to our community and we hope to grow the viewing audience in 2018. We also held our second blog carnival at signalsblog.ca on the controversial 'Right To Try.' Both are still online.



THIS IS US

For the first time, CCRM has a corporate video that captures (much of) what we do. Visit ccrm.ca (Media Centre/RM Network Videos) to see our facilities, hear from partners and appreciate how we're working with the community to support and advance the field. The patient who shared her story reminds us what we're all working towards. Have a look!



WE HELD A CELEBRATION

Grand opening, open house, ribbon-cutting... whatever you call it, we had ours in September. Government, industry and academic partners spoke at our event to recognize where we've come from and where we're going. A few hundred guests enjoyed the unveiling of a bronze statue of Drs. Till and McCulloch and later guests had the opportunity to meet and mingle.



INDUSTRY NETWORKING EVENTS

From the Translation Academy at Cell & Gene Therapy World in Miami to a speed networking event at the Till & McCulloch Meetings, we hosted several unique industry events that got people talking. The evening at Torgy in Toronto featured an art installation – the Cell Cave – that fully immersed the viewer in the experience and gave people lots to discuss.

BY THE NUMBERS:

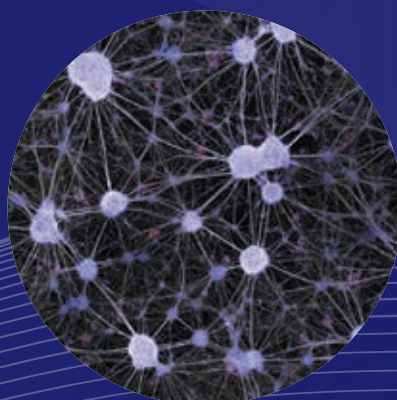
CELLS I SEE

2 WINNERS:
SUPERNOVA TANGO FOR
PEOPLE'S CHOICE AND
THE MATRIX FOR THE
GRAND PRIZE

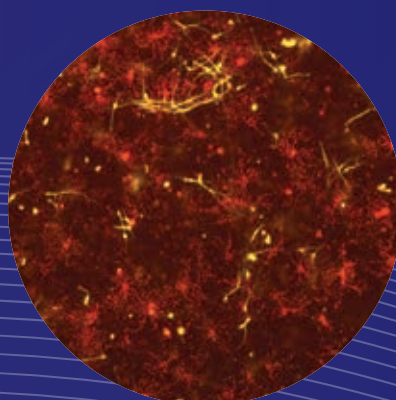
27 SUBMISSIONS
(A RECORD SINCE CCRM
TOOK OVER RUNNING
THE CONTEST IN 2014)

1,330 VOTES
CAST FOR PEOPLE'S
CHOICE CONTEST

700,000
(TO MID-NOVEMBER)
ORGANIC IMPRESSIONS
ON TWITTER

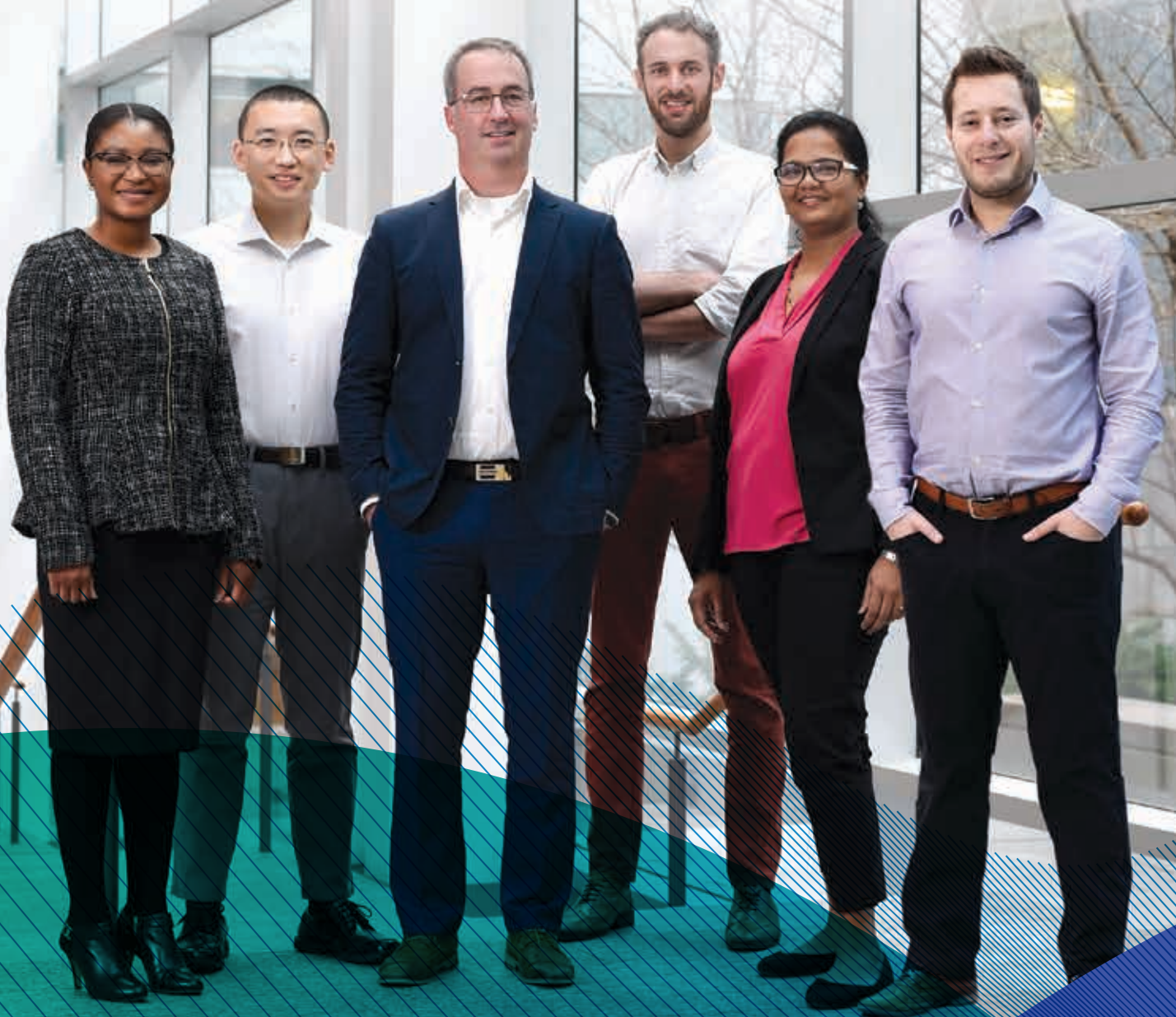


The matrix by Qin Liang



Supernova Tango by Marissa Lithopoulos

A NEW CANADIAN FACILITY FOR DELIVERING CELL AND GENE THERAPIES IS OPENING SOON



CCRM's cell therapy manufacturing capabilities have expanded significantly with the progress of the "Deliver" business unit. This team's focus is on providing a Good Manufacturing Practices (GMP) facility offering services and quality management systems to clients in partnership with the University Health Network (UHN).

Manufacturing a cell therapy product to be used in human clinical trials necessitates contamination-free manufacturing techniques, otherwise known as aseptic manufacturing, which require a specialized containment strategy. Physical environment, systems, procedural and personnel controls provide the foundations for this "clean" setting. These controlled conditions are dictated by the marketing authorities (Health Canada, U.S. FDA, EMA, etc.) regulating and approving regenerative medicine therapies and products. A GMP facility provides the clean environment that allows for open manufacturing, which also requires highly skilled personnel to be involved.

Thanks to financial support provided by the University Health Network (UHN) through a Canadian Foundation for Innovation (CFI) grant for construction, equipment and operations, and with contributions of its own, CCRM is building and will operate the Centre for Cell and Vector Production (CCVP) GMP facility.

The CCVP will be located at CCRM in downtown Toronto, adjacent to hospitals and research institutes. It will be managed by CCRM's Deliver team – expected to grow to 30 personnel – when it is operational this year. It is designed to the current and future technologies that are driving this

industry forward, allowing CCRM to deliver regenerative medicine and cell and gene therapy products for early phase clinical trials for years to come. CCRM will also assist with the transfer to clients' manufacturing facilities or to a contract manufacturing organization (CMO) when their early clinical trials prove successful.

Steven Keizer, Senior Quality Assurance Manager at CCRM, is leading operations for the Deliver team.

"The CCVP will be a leading-edge facility providing a clean room environment, cutting-edge quality assurance and control processes, and highly qualified personnel," says Steven. "This facility and its services will provide companies with the optimal setting to have their regenerative medicine products manufactured to run their Phase 1 and 2 clinical trials."

The 14,000 ft² (~1,300 m²) facility will be compliant with Health Canada and its counterparts in the U.S. and Europe. It will feature eight cell therapy suites and two vector production suites. Services will include contract manufacturing, cell banking, fill and finish, technology transfer, assay development, process development and GMP training.

CCRM's Deliver team is also helping to operate the Sunnybrook Research Institute's (SRI) GMP facility and sharing our GMP quality management systems. In return, CCRM will be able to offer services out of the SRI GMP facility beginning this spring.

"From a strategic perspective, CCRM's GMP offering to clients will mark a significant advancement for both us and the industry," says Steven. "As a critical component in the cell therapy manufacturing process, access to this facility provides an opportunity for companies, researchers and non-profit organizations to take the steps necessary toward regulatory approval of their cell therapies, contributing to progression of therapies from the bench to the bedside."

While assisting companies with meeting their goals, the completion of our GMP facility will allow CCRM to help the industry move one step closer to truly delivering on the promise of regenerative medicine as a possible cure for many diseases and conditions.

PROFILE: DR. ANDRAS NAGY

CREATING FAIL-SAFE STEM CELL TREATMENTS



Dr. Andras Nagy MD, PhD is a Senior Investigator at the Lunenfeld-Tanenbaum Research Institute at Mount Sinai Hospital in Toronto. He is also a member of CCRM's Founders Advisory Board. Dr. Nagy is one of the world's leading regenerative medicine researchers focused on stem cells and tissue engineering, and his discoveries have helped to advance the global regenerative medicine field immensely.

In 2005, Dr. Nagy helped to establish Canada's role as a leader in regenerative medicine by creating the country's first human embryonic stem cell lines. Four years later, he discovered a new, non-viral method of making stem cells from other cells in the body using reprogramming transgenes, with the potential to cure spinal cord injuries, macular degeneration, diabetes and Parkinson's disease. It was another global first.

The year 2015 marked another important milestone when Dr. Nagy partnered with Dr. Armand Keating, of Toronto's University Health Network, to establish a new company called panCELLa. Based on Dr. Nagy's work in this area, panCELLa seeks to develop research and clinical grade genome edited stem cell lines, which provide a very high level of safety before and after the therapeutic cell derivatives are introduced into the patients. This solution is called the FailSafe system.

"The company is built upon a new, proprietary technology for genetic editing, which will lead to FailSafe cell therapies," says Dr. Nagy, Chief Scientific Officer of panCELLa. "panCELLa's objective is to help ensure, with high probability, that cells will not be able to develop or derive a tumour."

It was clear that in panCELLa's formative months, the company was rife with scientific expertise. What panCELLa needed was a partner who could provide the equivalent high calibre of business acumen necessary for the company to bring this discovery to market.

CCRM's experts in business development, technology evaluation and intellectual property (IP) were enlisted to provide support, working closely with Dr. Nagy and the rest of the panCELLa team. For example, CCRM provided input on panCELLa's business plan, and helped in the provisional and Patent Cooperation Treaty (PCT) phases of IP protection necessary to commercialize the technology.

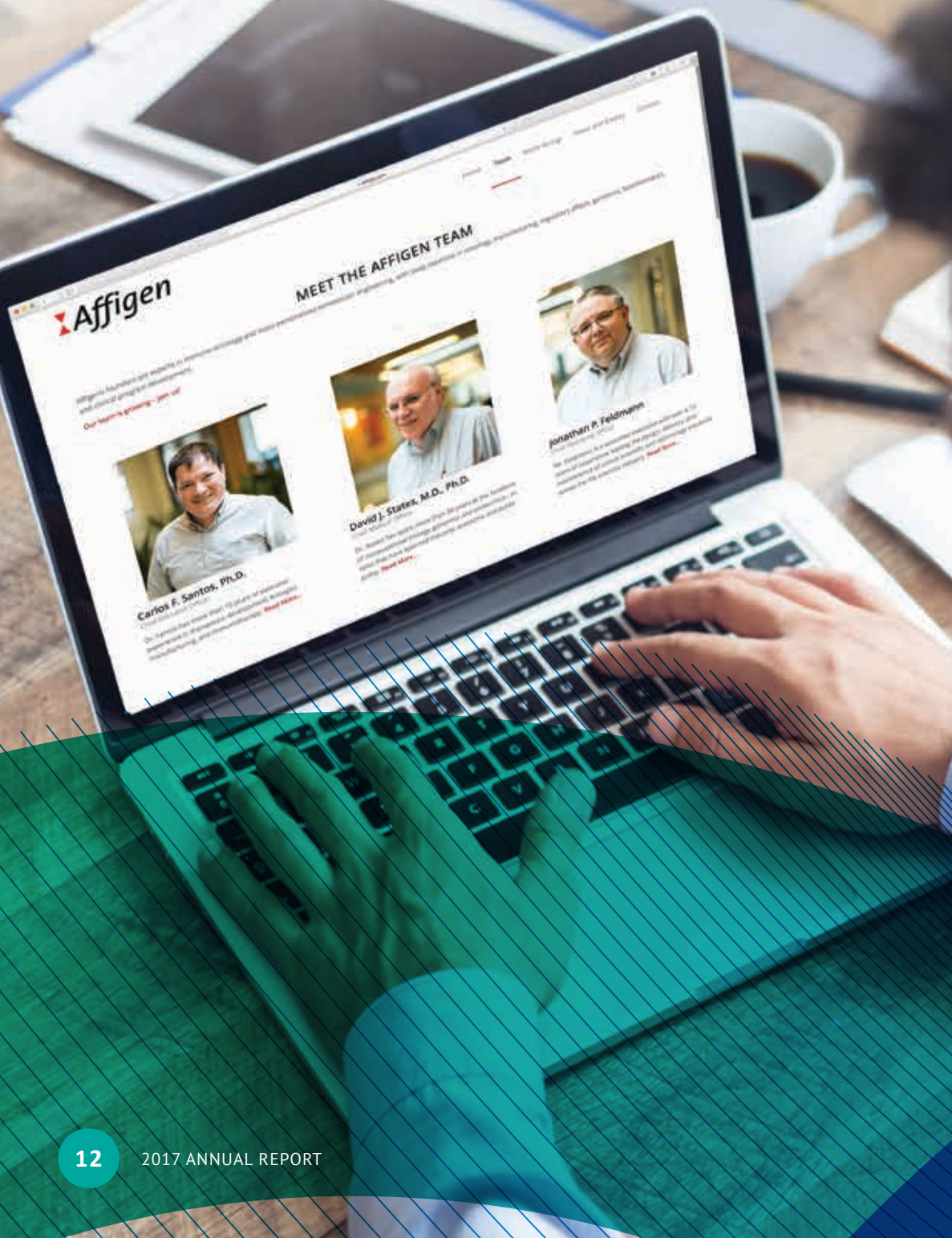
In 2018, Dr. Nagy reports that panCELLa will embark on securing Series A financing. Also, the company is considering the expansion of the availability of the proprietary technology through licensing agreements with other international companies that recognize the importance of safety in the cell therapy field.

"Ultimately, we want to create safe cell therapies in many disease areas, which would eventually be panCELLa's products," Dr. Nagy explains. "My research teams are now on the front lines and investigating different disease areas, including blindness, diabetes, arthritis, stroke and multiple sclerosis."

Dr. Nagy envisions that the relationship with CCRM will evolve in lock-step with panCELLa's trajectory.

"As panCELLa prepares for cell production in the future, we anticipate that the partnership will become stronger and more involved. We can utilize CCRM's Toronto facility to develop cell lines based on panCELLa's proprietary technology, bringing the company one step closer to producing safe treatments for patients," says Dr. Nagy.

AFFIGEN BRINGS ITS CANCER TREATMENT VISION TO REALITY BY LEVERAGING CCRM'S EXPERTISE



The cancer therapeutics of the future will be tailored to the patient and the tumour. A U.S. company, Affigen, is positioned at the forefront of developing these therapies. As the company embarks on this new frontier of medicine, they have enlisted CCRM for its expertise and technical skills.

Receiving a diagnosis of an incurable cancer, such as B-cell lymphoma or leukemia, can be devastating. Living with the effects of current treatments for these illnesses can also be difficult. This is because today's treatments do not discriminate between the different cells in the body, and healthy cells can be destroyed alongside the cancerous ones, resulting in cellular damage and toxicity.

Understanding the patient's experience, Affigen faces this scenario head-on. The company, founded in 2016 and based in St. Louis, Missouri, is made up of experts with over two decades of experience in developing immunotherapies for cancer.

Affigen's solution: Make a truly custom-made therapeutic that targets the proteins that distinctly brand a cancer cell in each patient.

"Affigen pioneers a new era of creating bespoke cancer treatments for individual patients," says Carlos Santos, PhD, CEO of Affigen. "By exclusively focusing on the proteins that are unique to cancerous cells, we can produce therapies that eradicate these cells while saving other cells in the body. With this precise approach, both toxicity and damage to healthy cells can be avoided, resulting in better outcomes in a safer manner."

Affigen has chosen to work with CCRM to support the development and commercialization of a closed platform to produce individualized, tumour-identifying therapeutics.

"We develop therapies that advance the field in terms of rapid protein engineering, drug discovery, cell culture systems, purification systems, and highly-consistent, automated batch release. Our processes bring rapid engineering of proteins down to an individual patient scale," says Dr. Santos. "By providing the expertise and equipment necessary to find solutions to these challenges, CCRM enables Affigen to develop these processes in a very efficient manner."

Affigen works with CCRM's Advance business unit, comprised of specialists in cell reprogramming and engineering, technology translation, and protocol development. The team works with academic and industry clients who focus on regenerative medicine, and cell and gene therapies, to progress technologies and treatments to a level of clinical utility.

Dr. Santos acknowledges that the partnership with CCRM is an asset to Affigen.

"CCRM is well-positioned with trained scientists who can bring extensive cellular therapy development expertise to Affigen's projects, and with an array of instrumentation to support the work and evaluate different experimental approaches. Why would we rebuild the wheel if CCRM already has it available? A major part of our process and technology development is now being done at CCRM."

Looking forward, Affigen will continue to build upon everything that's been learned in cancer immunotherapy and regenerative medicine as the company explores the development of patient-centric cancer therapies for illnesses beyond B-cell lymphomas and leukemias. Although these therapies may take different forms, the tailored treatment paradigm will remain.

"A world without cancer is our vision," shares Dr. Santos. "If we can develop the treatments that allow patients to truly and permanently get rid of cancer cells in their body without affecting healthy cells, that's our holy grail."

FEATURED STAFF

TRANSFORMATIONAL LEADERS



L-R Jonathan Yeh, Louise Shap, Mitch Sivilotti and Jana Machan

Jana Machan

MBA, MSc, VP Commercialization

Jana brings strategic and operational business experience based on her nine years as an executive at Baxter Canada, including establishing the Innovation and Market Access functions, leading Medical Affairs and steering a turn-around in Baxter's Renal Therapy business. At CCRM, Jana is focusing on developing new customer operations processes, expanding the client base and securing industry partners to further augment CCRM's capabilities to drive breakthroughs in regenerative medicine.

Louise Shap

LLB, MHSc, General Counsel

Louise is a lawyer with over 15 years of experience working in the health care sector. Louise has worked at the Ministry of Health and Long-Term Care, at Fasken Martineau as an associate in the health law practice group, as the assistant director of the legal department at Anapharm (a contract research organization), and in industry at Baxter and GSK. She now manages all legal affairs at CCRM.

Mitch Sivilotti

MSc, MFIN, Chief Operating Officer

Mitch, an experienced C-level executive, brings significant scientific, entrepreneurial and business management experience to CCRM. He has founded several companies over the past decade, most notably TotipotentRX, a company focused on cell-based clinical trials in cardiovascular disease. An entrepreneur at heart, Mitch was attracted to CCRM by the rapid development and commercialization of technologies/therapeutics where he provides hands-on guidance to company spin-out teams.

Jonathan Yeh

PhD, MBA, Manager, Venture Development

Jonathan is leading operations for CCRM's Launch business unit, which is focused on company creation and investment in regenerative medicine. Jonathan focuses on overseeing operations and driving forward CCRM Incubation Teams (read more about them on the next page) and refining the unit's investment intake pipeline. Coming from a background of science and business, Jonathan blends together training in stem cell research and drug development with practical experience in diagnostic and device commercialization strategy.

STRATEGIC COMPANY CREATION WILL BUILD CANADA'S REGENERATIVE MEDICINE ECOSYSTEM OF THE FUTURE

Launching new companies has consistently been part of CCRM's plan to help grow the regenerative medicine ecosystem, but this takes time. At CCRM, we aim to bring a critical mass in regenerative medicine to Canada. By strategically launching the Canadian anchor companies of tomorrow, we will form a network of product developers, service providers and foundational technologies that will grow the industry and attract the best leaders, companies and capital here.

To a large degree, commercial successes from innovation hubs such as California, Boston and Israel are buoyed by the critical mass of technology, leadership and capital focused at these sites, and boosted by a collaborative collegiality among emerging spin-offs. It's not uncommon for therapeutics companies to set up shop in any of these regions and partner with local service providers who in-license technologies from local technology companies.

CCRM is taking a proactive role to capitalize on opportunities and replicate the innovation hub model in Canada. We are sourcing the best bundles of technology to identify therapeutic, enabling and tech companies and matching them with market needs through our "incubation teams" initiative. Multi-disciplinary groups of scientists, engineers and business professionals, from across all units at CCRM and its networks, make up the incubation teams, who are looking for market gaps and pairing them with promising regenerative medicine technologies. We currently have three teams driving forward two therapeutic start-ups and one enabling concept.

These small teams are meant to function like start-ups: lean, nimble and with rapid decision-making authority. However, unlike most start-ups, these teams have the advantage of leveraging the scientific expertise, infrastructure, and years of industry leadership found at CCRM. The incubation teams nurture entrepreneurship efforts and offer unique training for our employees with the opportunity for them to become champions within an exciting start-up environment.

Once a product or business model reaches a point of operational proficiency and attractiveness to investors, it will be partnered with experienced leadership from CCRM's entrepreneur network, supported by an investor syndicate and launched.



THE FUTURE STARTS HERE

TECHNOLOGY DEVELOPMENT AT CCRM

35 BILLION PLURIPOTENT
STEM CELLS PRODUCED IN AN 8 L
BIOREACTOR CULTURE

8 BILLION
CARDIOMYOCYTES PRODUCED

OVER **\$2 MILLION** IN
CONTRACT SERVICES DELIVERED

LENTIVIRAL PRODUCER
CELLS SCALED UP TO A
**50 L STIRRED TANK
REACTOR**

23 iPSCs REPROGRAMMED

15+ INTERNAL DEVELOPMENT
AND TECH EVALUATION PROJECTS
RUNNING IN 4 CORE FOCUS AREAS

5 iPSCs GENE CORRECTED

3 INTERNAL DEVELOPMENT
COMPANIES INITIATED

IMPACTING KNOWLEDGE AND EXPERTISE ON A GLOBAL SCALE

In addition to the tried and true training and education events that we run every year – such as the Cell and Gene Therapies workshop that follows the Till & McCulloch Meetings, for example – 2017 included some new initiatives that were a hit and will likely be repeated in the future.

The organizers of Cell & Gene Therapy World (January, Miami) asked us to co-lead their Translation Academy by recommending programming and inviting speakers. With an emphasis on global translational innovation models, CCRM's partners in Australia, Israel, Japan, the U.K. and the Netherlands took to the stage to inform the mostly industry crowd of the advances and capabilities happening in their countries. A panel entitled "Europe: caught between a Trump and a Brexit" was an amusing and engaging way to end the first day.

In partnership with Medicine by Design and the Rotman School of Management at the University of Toronto, CCRM was pleased to help organize and host a new training initiative for local and international trainees. Summer by Design welcomed 12 emerging regenerative medicine leaders from universities in Australia, Singapore, Japan, Germany, the Netherlands, Sweden and the U.K., and two from the University of Toronto. The month-long program included attending two conferences, meetings with start-ups, venture capitalists and the technology transfer office at the Massachusetts Institute of Technology, three intensive weeks of lectures and group work on business aspects of regenerative medicine taught by CCRM staff, lab visits at the local hospitals and the University of Toronto, presentation skills training and more. The hope is to expand

the program to more local trainees and also provide opportunities for Canadian students to be hosted abroad in a similar program.

One year ahead of schedule, CCRM hosted the Business of Regenerative Medicine: Leadership, Innovation and Entrepreneurship (BRM 2017) in Toronto. For our second time at bat hosting this event that is run in partnership with Case Western Reserve University, Harvard Stem Cell Institute and Georgia Tech, CCRM co-hosted with the Rotman School to offer some new sessions and a pitch competition for start-ups. Our largest audience to-date was treated to talks from industry leaders from Canada, the U.S. and U.K., and delegates benefited from investors' feedback to entrepreneurs pitching their companies.

BioCanRx, CCRM, CellCAN, Foundation Fighting Blindness, OICR, OIRM and Stem Cell Network partnered on its first event for trainees: Bench to Bedside for Biotherapeutics (B3). The one-day event focused on how to translate research to take it to the clinic. Topics included recognizing if your discovery is translatable, manufacturing considerations, designing pre-clinical studies to support in-human trials, reimbursement and more. The Toronto event was videotaped and will be shared through the partners.

Some key invited talks/presentations/workshops included:

- CCRM and Stem Cell Network held a workshop to address regulatory gaps of minimally manipulated autologous cell therapies for homologous use in Canada. See the report in the September issue of *Cytotherapy*.

- Several employees participated in RegMedNet webinars.

- Dr. Calley Hirsch discussed "Identification of a high titer lentiviral production alternative to facilitate RM therapies" for Thermo Fisher's 24 Hours of Stem Cells.

- Various staff presented at OIRM and Medicine by Design symposia and workshops throughout the year, and to student groups at the University of Toronto.

- Dr. Lise Munsie presented "Genome engineering to introduce a fluorescent reporter into human pluripotent stem cells to study cardiac disease" at ISSCR in Boston.

BUILDING NEW PEAKS OF EXCELLENCE IN REGENERATIVE MEDICINE

Medicine by Design is a regenerative medicine initiative at the University of Toronto that undertakes transformational research to improve health outcomes and create value.

Medicine by Design works closely with CCRM to identify and plan translational pathways for its research discoveries. The two organizations are co-located, along with the Ontario Institute for Regenerative Medicine (OIRM), on the same floor in Toronto's MaRS Discovery District, creating a globally competitive hub for regenerative medicine research, translation and commercialization.

Highlights from Medicine by Design's second year include partnering in the hiring of five new faculty members and awarding \$2.5 million in new funding through its New Ideas and Post-Doctoral Fellowship awards. In collaboration with CCRM and University of Toronto's Rotman School of Management, Medicine by Design also launched Summer by Design, a program for graduate students and post-doctoral fellows from partner universities around the world who are interested in building expertise in regenerative medicine translation and commercialization.

Structured like a design studio, Medicine by Design brings together 110 scientists, engineers and clinicians from across University of Toronto and its affiliated hospitals to conceive, create and test strategies to address critical problems in regenerative medicine. Converging across disciplines, these researchers generate and use emerging methods such as genome

editing, computational modelling and synthetic biology to deepen understanding of core biological concepts and devise new therapeutic approaches.

Building on decades of made-in-Toronto discoveries, Medicine by Design is developing new peaks of excellence and strengthening Canada as a global leader in regenerative medicine thanks to a \$114-million investment from the Canada First Research Excellence Fund. Its activities include:

research
awards

faculty
recruitment

clinical
translation

knowledge
mobilization

commercialization

international
partnerships



UNIVERSITY OF
TORONTO



FUNDED RESEARCH TO BUILD CLINICAL AND COMMERCIAL CAPACITY IN ONTARIO

The Ontario Institute for Regenerative Medicine (OIRM) is a non-profit stem cell institute funded by the Ontario government and dedicated to transforming discoveries into clinical trials and cures. Through its commitment to collaboration and partnerships, OIRM leverages its resources to fund and support promising advances and is a passionate champion for investigators and their patients in building a healthier future for Ontario, Canada and the world.

OIRM and CCRM are partners in advancing regenerative medicine research and innovation. In late 2016, one of OIRM's funded Disease Teams, led by Dr. Michael Laflamme at the University Health Network, became a foundational platform for BlueRock Therapeutics and is being advanced in collaboration with CCRM. In 2017, CCRM and OIRM also worked together to engage research groups in five major centres in Ontario and in providing commercialization expertise at workshops and symposia organized by OIRM.

OIRM's Disease Teams are large, collaborative research projects representing areas of strength in Ontario with the potential for clinical and commercial advancement. In 2017, OIRM funded five Disease Teams in the areas of heart regeneration, brain repair, septic shock, lung injury and vision repair. One of these Disease Teams includes a clinical trial and three others are in the late preclinical phase.

Building capacity in Ontario to ensure continued strength and excellence in stem cell and biotechnology research also involves solving problems in the development pipeline. OIRM's New Ideas research program provides support to achieve this by providing targeted funds to answer key research questions. In 2017, OIRM partnered with Medicine by Design to fund 12 New Ideas projects that are seeking to develop new research insights and technologies, such as improved methods to grow cells and tissues, mathematical models of evaluation, or platforms that could improve therapies for a range of conditions such as autism spectrum disorder, osteoporosis and lung disease.



KICKSTARTING THE FUTURE

BUSINESS DEVELOPMENT EFFORTS AND
SUPPORTING CANADIAN START-UPS

\$1.8 MILLION INVESTED
TO-DATE IN CCRM'S PORTFOLIO
COMPANIES

80 DISCLOSURES
RECEIVED AND REVIEWED

25 PITCHES TO ENCOURAGE
COMPANIES TO CHOOSE CANADA
FOR THEIR CLINICAL TRIALS

**17 REGULATORY
PROJECTS** EXECUTED
FOR EXTERNAL AND INTERNAL
CLIENTS

**15 INVITED PUBLIC
TALKS** OR PANELS

7 PUBLICATIONS

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



PORTFOLIO AND ASSOCIATE COMPANIES



KisoJi



COLLABORATIVE PARTNERS



INDUSTRY CONSORTIUM

CCRM has established a consortium of 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.

ADVISORS

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ACCOMPLISHING KEY MILESTONES FOR CELL AND GENE THERAPY CLINICAL TRIALS

- ▶ Construction of the CCVP is underway!
- ▶ The Deliver team has manufactured clinical-grade cells for two Phase 1 trials. One patient has passed the nine-month mark with good safety data.
- ▶ Wondering where to do your cell manufacturing? Deliver and Build team members have submitted a paper comparing regulatory requirements and advantages of the big four market authorities (Health Canada, U.S. FDA, EMA and Japan's Pharmaceuticals and Medical Devices Agency).

CCRM

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