FUTURE-READY

2023 ANNUAL REPORT

CCRM

Commercializing
Living Therapies

CCRM's mission is to generate sustainable health and economic benefits through global collaboration in cell and gene therapy, and regenerative medicine.

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 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 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.

Contents

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

The CCRM Team

December 2022

EXECUTIVE MESSAGE

Ready, and excited, to face the future

CCRM's senior leadership team

The passing of a new year, like an annual report, is an excellent time to reflect on what a person, or organization, has accomplished and whether it has met its strategic goals and objectives – or resolutions – in the past 12 months. This year we accomplished a goal that was set in 2011 when we launched: CCRM needed to be self-sustaining by the time its government funding ended. We are extremely proud that we have achieved that milestone.

CCRM was funded in 2011 as a centre of excellence for commercialization and research through the Government of Canada's Networks of Centres of Excellence (NCE) program. Our funding ended March 31, 2023. We leveraged $15 million from the NCE in 2011 and another $15 million in 2018 into over $350 million to develop a skilled team (about 500 current and former employees), specialized infrastructure, 20 companies (leveraged by over $1 billion in investment), and the academic, industry and investor networks we have today. Although we may choose to apply for government funds for specific projects in the future, we are now operationally independent. Establishing and launching OmniaBio Inc., in March 2022, helped to make that possible.

It is not an exaggeration to say that CCRM would not exist without the funding and support of the NCE. We are truly grateful to the Government of Canada for having confidence in CCRM's ability to bridge the gap between academia and the marketplace, and to support us again in 2016 with new funding as we embarked on our ambitious efforts to build manufacturing capabilities for Canadian and international cell and gene therapy developers. We believe the government's faith in us was well-placed as Canada will reap the rewards from a stronger regenerative medicine sector that generates economic benefits through new jobs, keeping and attracting intellectual property in Canada, and improving patient health.

OmniaBio is a subsidiary of CCRM. It is on track to be one of North America's largest cell and gene therapy CDMOs when its second site is completed in 2026-7. The first site, at McMaster Innovation Park in Hamilton, Ontario, is 120,000 square feet and it will be completed in mid-2024. The building will have 17 clean rooms and lab space for process and analytical development, manufacturing science and technology (MSAT), and quality control.

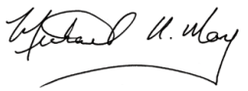
OmniaBio is expanding on the preclinical and early-phase capabilities developed at CCRM to provide customers with Good Manufacturing Practices (GMP)-compliant manufacturing all the way to the commercial stage. CCRM anticipates that OmniaBio will help make Canada a leader in cell and gene therapy manufacturing and raise Canada's profile on the world stage. Read more about OmniaBio on pages 8-10.

With OmniaBio being led and operated by a highly competent team of seasoned professionals, CCRM can focus on industrializing manufacturing, venture investment, technology development, growing a skilled workforce and establishing international collaborations through hubs modelled after CCRM in Canada. In 2023 we were pleased to add a second CCRM hub, this time in Sweden. CCRM Nordic is backed by funding from Vinnova, Sweden's Innovation Agency. It has an impressive Board of Directors and a strong industry consortium that includes AstraZeneca, CombiGene, Cytiva, Getinge, Takara Bio Europe, TATAA Biocenter and Verigraft, as well as local innovation actors GoCo Health Innovation City and GU Ventures. Read more about CCRM's hubs on page 13.

Back in Canada, CCRM and OmniaBio launched a spin-off called LineaBio Inc. to offer customers GMP-grade, regulatory-compliant induced pluripotent stem cell lines employing a new model for accessing GMP cell banks that is faster and less expensive, and includes analytics for regulatory filings. CCRM will announce more new companies this year that have matured through our successful incubation program.

With almost 2,000 clinical trials in progress as of December 2023, according to the Alliance for Regenerative Medicine, there remains huge demand for manufacturing and a need to keep pushing forward to optimize processes and bring down costs. CCRM, along with OmniaBio, and partners such as Cytiva, will continue to innovate to meet the needs of developers.

We are excited by the opportunities we see in the cell and gene therapy field and remain committed to revolutionizing health care by solving the big problems in regenerative medicine – to prepare for the future.



Michael H. May, PhD
President and CEO

2023 Worth Noting

New corporate video

Lights, camera, action! In case you missed it, CCRM kicked off 2023 with a new corporate video. Although we already have an award-winning video from 2017, the organization has grown substantially since then. The new video contains information about OmniaBio Inc., our subsidiary; CCRM Enterprises, our investor arm; Notch Therapeutics, our first incubation company; and much more. You can watch the video here.

CCRM, CATTI and OmniaBio join HI³ research hub

Early in 2023, the Government of Canada funded five research hubs at universities across the country to bolster research and talent development efforts led by the institutions, working in collaboration with their partners. The hubs combine the strengths of academia, industry and the public and not-for-profit sectors to jointly improve pandemic readiness and the overall health and well-being of Canadians. CCRM, CATTI and OmniaBio are stakeholders in the University of Toronto-led Canadian Hub for Health Intelligence and Innovation in Infectious Diseases (HI³) and participants in three additional hubs.

Public-private partnership leads government's Biologics Manufacturing Centre

In April, the National Research Council of Canada and CCRM announced that the not-for-profit corporation Biologics Manufacturing Centre (BMC) Inc. had officially assumed responsibility for the operations of the Biologics Manufacturing Centre in Montreal, Quebec. The contract manufacturing organization will produce drugs for rare diseases and be prepared to pivot to support Canada's response to any future public health emergencies. Since then, Isabelle Caron has been named CEO and a new Board of Directors was recently announced.

2023 CELLS I SEE ART CONTEST

People's Choice Award Winner

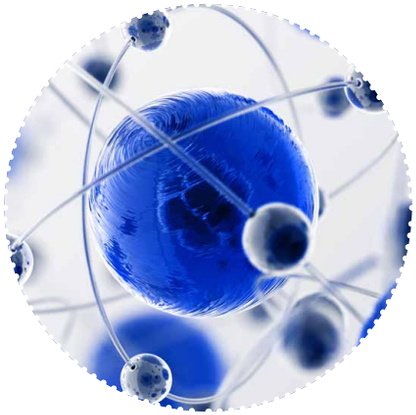
Congratulations to Iram Fatima Siddiqui, McGill University, winner of the People's Choice for "Bubbles, bubbles everywhere. Be as bubbly as your fat cells."
The People's Choice is awarded to the entry with the highest number of likes on Facebook.

Next Great Big Ideas Summit and Podcast

CCRM and OmniaBio Inc. were invited to participate in the inaugural Next Great Big Ideas Summit, held in Hamilton, Ontario, in October. The full-day event featured life sciences leaders, disruptors and innovators from across Canada, and proceeds went to support the McMaster Children's Hospital. Michael May, President and CEO of CCRM, and Mitchel Sivilotti, CEO of OmniaBio, were also interviewed for the NGBIdeas Podcast, along with other leaders. You can listen to the podcast to hear from Canada's visionaries and leaders.

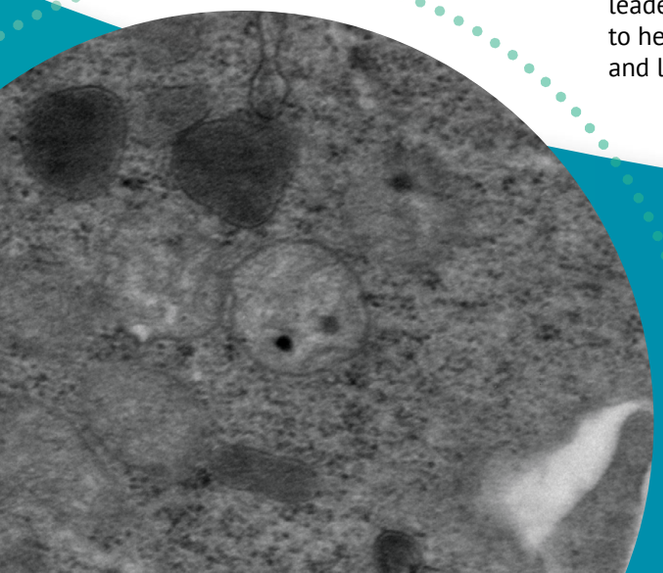
Awards

Congratulations to our award-winning leaders. Michael May, President and CEO, is the recipient of the 2023 Le Prix Luc Sensebé Innovation and Leadership Award from the International Society for Cell and Gene Therapy. Peter Zandstra, PhD CM, Chief Scientific Officer at CCRM and Director, School of Biomedical Engineering, University of British Columbia, has received the inaugural Scientific Entrepreneurship Award from Life Sciences British Columbia.



A blog worth reading

Based on a review of thousands of blogs on the web and ranked by traffic, social media followers and freshness, Feedspot named CCRM's CGT Manufacturing blog one of its top gene therapy blogs. The CDMO Education Centre blog provides tips and insights on commercialization and working with a contract development and manufacturing organization, like OmniaBio Inc. Since the launch of OmniaBio, the blog has had a new home. Please check it out and subscribe as new content is being added. (Please note that many posts have been archived.)



2023 CELLS I SEE ART CONTEST

Grand Prize Winner

Congratulations to Charlotte Ling, University of Toronto, Grand Prize winner for "Mad-ochondria." This was the entry with the most votes from delegates at the annual Till & McCulloch Meetings.

OMNIABIO

Bringing maturity to cell and gene therapy

A subsidiary of CCRM, OmniaBio Inc. is a contract development and manufacturing organization (CDMO) focused on cell and gene therapies. Benefitting from CCRM's existing expertise, OmniaBio provides a continuum of advanced process and analytical development, and manufacturing capabilities, by enabling dedicated support for clients from early clinical phase to commercial supply. Founded in 2022, OmniaBio holds extensive expertise in cellular immunotherapy, lentiviral vectors (LVVs) and induced pluripotent stem cells (iPSCs).

Located between Toronto and the U.S. border, OmniaBio's new commercial manufacturing site—currently mid-construction—will anchor OmniaBio as the largest cell and gene therapy (CGT)-focused CDMO in Canada and will open with commercial capacity in 2024 in a site totalling 120,000 square feet. A second facility, totalling 315,000 square feet, is planned to open in 2026.

New website and video

During the busy fall conference season, OmniaBio launched its new website. With a fresh design, graphics and updated copy, OmniaBio's new website reflects its unique value proposition, services and commitment to innovation, while also showcasing how it supports its clients. The new site shares resources, features a blog, and keeps visitors updated on relevant conferences to attend in the cell and gene therapy field. And because OmniaBio has a large hiring mandate, the Careers page is a popular first stop.

A new drone video about OmniaBio provides a bird's eye view of the facility and its strategic location in Hamilton, Ontario, close to two essential services: Hamilton International Airport, Canada's largest overnight express cargo airport, and Pearson International Airport for passengers. Hamilton also offers a perpetual wealth of talent graduating from McMaster University and Mohawk College. The video takes us inside the massive space. Watch it here.

Highlights from OmniaBio in 2023

January

OmniaBio expanded its footprint, last January, by adding a new process and analytical development lab space at the McMaster University Medical Centre in Hamilton, Ontario. Acquiring additional lab space—including hiring more than 20 experienced CGT scientists—ahead of the opening of OmniaBio's commercial facility in 2024 enables better support to the company's growing portfolio of customers, accelerating their initiatives. The expanded lab space is complementary to the ongoing process and analytical development projects already taking place at the site in Toronto, Ontario.

A sneak peek of OmniaBio's new facility video. Watch it here.

March

In March, OmniaBio and Catamaran Bio Inc., a Boston-based biotechnology company developing off-the-shelf natural killer (NK) cell therapies to treat cancer, announced their partnership to develop and manufacture Catamaran Bio's allogeneic CAR-NK cell therapies for the treatment of solid tumours. OmniaBio, along with CCRM, is developing Catamaran Bio's CAR-NK cell therapy process at their Toronto site, where the process development lab is co-located with GMP-compliant clean room suites to offer a seamless transition between process development and clinical manufacturing. The OmniaBio-CCRM partnership enables highly adaptable and flexible end-to-end support for therapeutics developers like Catamaran Bio to move rapidly from development to the clinic and beyond.

May

OmniaBio announced a partnership with the Canadian Advanced Therapies Training Institute (CATTI), CCRM, and the University of Guelph, to launch CATTI's first in-person training site at the University. The training program, which commenced in summer 2023, caters to recent post-secondary graduates, industry professionals and companies growing their roster of clean room manufacturing and quality control staff. The CATTI partnership will create a reliable system to support OmniaBio's ability to onboard and advance the careers of biotech manufacturing specialists at the speed it needs to meet market demand.

July

Along with CCRM and CCRM Australia, OmniaBio signed a Letter of Intent with The University of Queensland to establish the Advanced Cell Therapy Manufacturing Initiative (ACTMI) at the St Lucia (Brisbane, Queensland) campus of the University, on July 28. OmniaBio and its partners will work with The University of Queensland to establish ACTMI as a prominent centre for advancing cell therapy process development, benefitting patients and contributing to the overall progress of the global regenerative medicine sector. The partnership represents a significant stepping stone for OmniaBio in expanding its technology development network and offering CGT CDMO services to the Asia-Pacific region.

November

One year following the initiation of construction of its first facility in Hamilton, OmniaBio celebrated the completion of the building's outer core and shell, alongside partners Multiplex Construction Canada and McMaster Innovation Park. The construction project was executed under the leading-edge integrated project delivery (IPD) method, meaning the building was quite literally built on a foundation of innovation—fitting for the site that will anchor Canada's centre of CGT manufacturing excellence. The occasion marked a significant milestone as OmniaBio gears up to open its first commercial manufacturing site this year.

OmniaBio site visit, McMaster Innovation Park

CCRM Enterprises fuels innovation in cell and gene therapies

"As in prior years, CCRM Enterprises played an important role in the support of early-stage companies developing transformative technologies."

Cynthia Lavoie
President and Chief Investment Officer, CCRM Enterprises Inc.

CCRM Enterprises Inc., CCRM's for-profit venture investment arm, creates value for regenerative medicine-based technology and cell and gene therapy companies by making early-stage investments while leveraging CCRM's sector expertise and specialized infrastructure.

"Although the regenerative medicine sector faced headwinds in 2023, adversity fuelled innovation," says Cynthia Lavoie, PhD, Chief Investment Officer, CCRM Enterprises Inc. "As in prior years, CCRM Enterprises played an important role in the support of early-stage companies developing transformative technologies. It is continually laying the groundwork for a resilient and impactful future in this ever-evolving landscape."

CCRM Enterprises was proud to join several dedicated investor syndicates in 2023, allowing it to continue funding world-class companies in the field. It currently has a portfolio of 15 companies (we've exited from two) that span a broad modality mix within regenerative medicine. Some notable successes include a significant partnership between portfolio company Aspect Biosystems and Novo Nordisk, valued at up to $650 million, to develop bio-printed therapeutics for diabetes and obesity. ExCellThera, a Montreal-based cell therapy company, is advancing its lead product through clinical phases, and is achieving improvements in key long-term clinical outcomes for patients suffering from blood cancers.

The CCRM Enterprises team had an active year making several follow-on investments into two of its portfolio companies: ExCellThera and Feldan Therapeutics. Further, CCRM Enterprises was integral in forming and seeding CCRM's spin-out companies, including LineaBio, a new company offering GMP-compliant induced pluripotent stem cell lines.

Dr. Lavoie also had a busy year on panels and giving talks. As an example, she engaged postdoctoral fellows and principal investigators at a workshop called "The Business of Regenerative Medicine: Entrepreneurship one stem cell at a time" at the 2023 Till & McCulloch Meetings. As keynote speaker, Dr. Lavoie discussed Canada's research and investment ecosystem for advanced therapies. Participants learned about intellectual property, scientific and clinical validation, financing, regulatory approvals, and more.

Looking to 2024, CCRM Enterprises, in collaboration with CCRM's scientists and engineers, will continue to investigate the newest advances in gene editing, and cell and gene therapy, to guide its disciplined capital allocation strategy. The team will also explore new funding models for Canadian companies while supporting the commercialization efforts of its existing portfolio companies. These initiatives will help bring treatments to market quickly, while achieving outsized returns.

Building a "visionary" future of health: The CCRM Foundation's impact on regenerative medicine

With two major milestones achieved, 2023 has been a pivotal year for The CCRM Foundation as it delivers on its mission of supporting the regenerative medicine ecosystem.

Supporting Visions of Science

In November, The CCRM Foundation launched its first community initiative by partnering with Visions of Science to support under-represented, racialized youth from low-income communities in the Greater Toronto Area with new and increased opportunities for access to science, technology, engineering and mathematics (STEM) education.

Visions of Science proactively engages youth at critical stages of development and facilitates year-round STEM learning opportunities to strengthen support networks and ensure equitable STEM education and academic and career pathways. This approach aligns with The CCRM Foundation's efforts to support education and training to open doors to the world of regenerative medicine.

The CCRM Foundation, CCRM, its subsidiary OmniaBio Inc., and its partner, Medicine by Design, will build a robust partnership consisting of financial support totalling $75,000, as well as opportunities to co-create hands-on experiences for youth to come face-to-face with scientists and their work. This commitment will support Visions of Science over three years.

"Visions of Science is proud to be launching a partnership with The CCRM Foundation that will support a pathway of STEM learning and opportunities for Black and racialized youth from low-income communities," explains Eugenia Duodu Addy, PhD, CEO, Visions of Science. "This will contribute to life-changing outcomes for youth and their communities, and promote a STEM future that is more innovative, sustainable, and ultimately more equitable for all."

Expanding its network

The CCRM Foundation has also deepened its network of partners, which encompasses Medicine by Design. They will work closely to identify significant opportunities to support high-risk, high-reward research that will drive academic-led innovation within cell and gene medicine.

In the year to come, The CCRM Foundation will engage another group of key stakeholders: CCRM's former employees, or "alumni." With a plan in development, alumni can expect to learn about opportunities to create impact and give back within the regenerative medicine ecosystem.

CCRM's Australian and Nordic hubs advance global collaborations in regenerative medicine

CCRM's global hubs mark a year of significant strides, driving a strategy that fosters collaboration and innovation on a worldwide scale.

CCRM Australia

Established in 2016, CCRM Australia has made many advances this year. Following incorporation as an independent, not-for-profit company limited by guarantee in 2022, CCRM Australia actively engaged with both government and the private sector in 2023, paving the way for potential projects across three Australian states – Victoria, New South Wales and Queensland.

Notably, CCRM Australia led a sector-wide bid for research funding under the Australian government's Cooperative Research Program, which will address challenges in developing advanced therapies. In collaboration with The University of Queensland as the lead research organization, a Cooperative Research Centre is proposed to undertake a 10-year, industry-led research program developing technologies in biomanufacturing, bioseparation, biomaterials and clinical enablement.

CCRM Australia's international relationships expanded this year, originating from attendance at key forums in Korea, China, Israel and the U.S.

CCRM Nordic

In May 2023, CCRM announced the formation of CCRM Nordic with the support of Vinnova, the Swedish Agency for Innovation Systems. CCRM Nordic, a not-for-profit based on CCRM's Canadian model, is backed by an industry consortium that will enable its specialized infrastructure and expertise focused on advanced therapy medicinal product (ATMP) development. It is well-positioned to help start-ups, researchers and investors across Sweden, the Nordic countries and Europe, in the translation and commercialization of technologies and therapies.

Fredrik Wessberg, CEO of CCRM Nordic, now oversees a team of 13 employees. They work with a Board of Directors with extensive knowledge and expertise in biotechnology and ATMPs.

In fall 2023, CCRM Nordic began construction of an interim process development lab and offices, to be operational in January 2024. The planning of its future process development and Good Manufacturing Practices facility in GoCo Health Innovation City, in Mölndal, Sweden, is underway, with a ground-breaking ceremony planned for spring 2024.

Looking forward, CCRM's global hubs will build on this year's momentum, powering innovation, propelling the commercialization of promising discoveries, and contributing to the growth of the sector in their own regions, and beyond.

Michael May, centre, with members of the CCRM Nordic team when they visited in fall 2023. From left to right, Jim Lund, Helena Choukair, Emma Johansson and Fredrik Wessberg.

Conceptual image of GoCo Health Innovation City, in Mölndal, Sweden, where CCRM Nordic will be housed.

Advancing Canada's regenerative medicine ecosystem with bespoke training opportunities across the commercialization pathway

CATTI: Accelerating CGT Workforce Readiness

As the cell and gene therapy (CGT) market grows, the industry needs qualified, skilled personnel to manufacture and produce CGTs following Good Manufacturing Practices (GMP). Recognizing the gap in highly qualified personnel (HQP), in 2021 CellCAN and CCRM launched the Canadian Advanced Therapies Training Institute (CATTI). By combining their strengths, CCRM and CellCAN, through CATTI, are growing the industry's manufacturing capabilities by offering specialized training to accelerate the market readiness of the workforce.

CATTI's training courses encompass a spectrum of critical themes in biomanufacturing excellence, including:

- GMP and Aseptic Processing
- Quality Management Systems, Good Documentation Practices, and Quality by Design
- Regulatory Compliance
- Risk Management, Quality Control and Product Release
- Manufacturing Techniques, Equipment and Handling

CATTI lab, University of Guelph

University of Guelph, Ontario Veterinary College

CATTI lab where in-person training occurs

In May 2023, CATTI announced its inaugural in-person training centre of excellence at the Ontario Veterinary College at the University of Guelph, continuing its mission of rapidly and efficiently upskilling Canada's biomanufacturing talent pipeline. The new site is the first stage of CATTI's larger, multi-site training strategy. This accomplishment was made possible thanks to a collaborative partnership between the university, OmniaBio Inc. and CCRM.

Focused on providing a highly specialized GMP training experience, the new site's program offers a combination of comprehensive and robust training courses both online and in-person. In the program's first year, its main emphasis has been on banking human pluripotent stem cells, perfecting aseptic techniques, optimizing GMP for CGT manufacturing, and honing laboratory skills — including pipetting, vessel handling and microscope operation, among other techniques.

The new training program's participants include recent post-secondary graduates, industry professionals and companies who are looking to build their clean room manufacturing workforce. The program will advance the competencies of biotech manufacturing professionals and enable companies to safely produce life-saving therapies. Over the next two years, CATTI is expected to train over 600 people, including more than 300 of OmniaBio's workers, a strategic customer of CATTI.

Take a virtual tour of CATTI's new state-of-the-art training facility

Click to watch

Recently, CATTI launched a five-day intensive human pluripotent stem cell (hPSC) GMP training course called Cell Therapy Bootcamp: hPSC Biomanufacturing. CCRM and OmniaBio informed and contributed insights to the bootcamp's content, whose aim is to enhance skills in maintaining hPSCs in culture within a simulated GMP environment.

The course, designed on industry-focused best practices, is arranged into five modules providing knowledge on each critical aspect for successful culture and maintenance of hPSCs. The five modules include: Introduction to hPSCs; Thawing and Seeding of hPSCs; Feeding and Observations; Passaging; and Harvest and Cryopreservation.

TMM, Cell Therapy Training Course and Micro-credentials

CCRM was pleased to support the Till & McCulloch Meetings (TMM) as a platinum sponsor this year and participated in the conference in a number of ways. Cynthia Lavoie, President and CIO of CCRM Enterprises, was the keynote speaker at a TMM pre-conference session "The Business of Regenerative Medicine: Entrepreneurship one stem cell at a time" tackling critical topics for entrepreneurs in the commercialization of a discovery. On the first day of the conference, CCRM's Glenn MacLean, Director, Technology Sourcing and Venture Development, led a breakfast session called "Experimental De-risking for Venture Creation" to outline the important differences in experimental design between academic research and supporting the commercialization of technologies. It was a "must-attend" for researchers with entrepreneurial interests.

A popular session at TMM is hearing the first-hand experience of a patient and this year we heard from Andre and Nathan Cordeiro, twin brothers living with an inherited disorder called Leber congenital amaurosis (LCA). This progressive condition had no treatment until gene therapy Luxturna came on the market. In 2022, the brothers were the first children in Canada to receive it. The results have been life-changing. CCRM was pleased to support this important session.

This fall, the International Society for Cell and Gene Therapy (ISCT) and the American Society for Transplantation and Cellular Therapy (ASTCT) hosted the Cell Therapy Training Course (CTTC). The CTTC is an application-based full-tuition program designed to train and develop future CGT leaders. CCRM and OmniaBio were pleased to support the CTTC as gold sponsors. This five-day, in-person course, occurring once every two years, combines lectures, discussions, mentoring and networking sessions led by elite CGT experts. OmniaBio's Sarah Lepage, Training Program Manager, audited the course, sharing best practices and bringing practical learnings back to CCRM and OmniaBio.

CCRM partnered with the University of Toronto's School of Continuing Studies and the Faculty of Applied Science and Engineering to offer three micro-credentials in biomanufacturing called Upskilling in Biomanufacturing. The unique, three-course program addressed GMP in biomanufacturing, quality assurance management, bioprocess analytics and optimization to improve job readiness and increase Ontario's biomanufacturing capacity. Students had the opportunity to meet with CCRM experts and tour our specialized facilities.

CCRM's Glenn MacLean

Christina Cordeiro with her sons Andre and Nathan

Portfolio Company Highlights

CLICK THE BOXES TO GET TO THEIR WEBSITES!

Through CCRM Enterprises, CCRM provides tailored support to advance the translation of promising discoveries from lab to market, and has supported the launch and growth of 20 companies that have gone on to raise over $1 billion. Here are some notable achievements from our portfolio companies in the last year.*

Aspect Biosystems

3D bioprinting of human tissues to build allogeneic therapeutics

Vancouver • Preclinical

Aspect Biosystems entered a partnership with Novo Nordisk worth more than US$2.6 billion to develop bio-printed tissue therapeutics for diabetes and obesity. Aspect has also continued to leverage its full-stack tissue therapeutic platform to advance its own therapeutic pipeline for metabolic and endocrine diseases. Aspect grew its team and doubled its footprint, was named one of *Fast Company's* "Next Big Things in Tech," and was awarded "Company of the Year - Scale" and "Gamechanger - Ambition" by the BC Tech Association.

Endogena Therapeutics

Regenerative molecules for retinal degenerative disease

Toronto/Basel • Clinical

Endogena Therapeutics' lead program targets retinal degeneration due to retinitis pigmentosa (RP), a group of inherited eye diseases that often leads to blindness. In February 2023, their experimental therapy EA-2353 was granted Fast Track Designation by the U.S. Food and Drug Administration (FDA) to support and expedite its development in this rare disease.

Endogena confirmed the program's concept in a genetic mouse model of RP, and the first in-human clinical trial in the U.S. enrolled ahead of schedule and has shown that EA-2353 is safe and well-tolerated.

Exacis Biotherapeutics

Messenger RNA reprogramming platform for engineered T and NK cells

Boston

Exacis Biotherapeutics' global immuno-oncology platform was acquired by Eterna Therapeutics (Cambridge, MA), which focuses on therapies created using mRNA cell engineering technologies. Exacis' platform of induced pluripotent stem cell (iPSC)-derived cell therapy candidates, created with mRNA cell reprogramming and mRNA gene editing, will be leveraged by Eterna to develop engineered cell therapies containing genomic edits, which are designed to enhance effectiveness. Exacis' CEO, Greg Fiore, will chair a new business development committee on Eterna's Board of Directors.

**Past exits: AVROBIO and Empirica Therapeutics*

ExCellThera

Create cell lines using molecules and bioengineering solutions to improve HSCT

Montreal • Phase 2

ExCellThera is a late-stage biotechnology company using novel blood stem cell expansion approaches to treat high-risk leukemias, myelodysplasias and other life-threatening diseases. The company's lead program, UM171 cell therapy, an allogeneic hematopoietic stem cell transplant (HSCT) for blood cancers, has been evaluated in over 100 patients and has successfully completed Phase 2 trials. ExCellThera was selected to give a data presentation in an oral session at ASH 2023 in San Diego, California, in December.

Feldan Therapeutics

Therapeutics using peptide-based shuttle technology

Quebec City • Preclinical

Feldan received two federal government grants this year to advance its programs. One will help develop the Feldan Shuttle formulations to treat skin conditions with unmet medical needs and support Feldan's pipeline expansion. The second will assist in the development of an intracellular delivery system for antisense oligonucleotides using the Feldan Shuttle technology to treat pulmonary diseases. Additional accomplishments include a Pre-Investigational New Drug application to the FDA for FLD-103, the incorporation of an Australian subsidiary, and participation in the 2023 Winter Clinical Dermatology Conference.

iVexSol

Manufacturer of stable lentiviral vector gene delivery technologies

Toronto/Boston • Commercially available

In 2023, iVexSol solidified its position as the leader in creating stable lentiviral vector (LVV) cell lines to solve the sector's LVV shortage. The company closed US$24 million in Series A-3 funding, signed a large biopharmaceutical collaboration, and released scientific data demonstrating the strongest performing LVV solution in cell and gene therapy. iVexSol also welcomed a newly promoted Chief Technology Officer and further expanded its Board of Directors and Scientific Advisory Board.

KisoJi Biotechnology

Single domain antibodies against hard-to-drug targets

Montreal • Preclinical

KisoJi made a breakthrough in its machine learning efforts: developing a unique mapping tool to enable visualization of all antibodies in a given library, enabling companies to see the totality of an immune response and prospectively screen antibody libraries for the desired utility rather than using traditional random and inefficient screening technologies. KisoJi's lead asset, a potent TROP2 antibody, was selected for a partnership with Cancer Research UK (CRUK), in which CRUK will design and fund the pending first-in-human studies.

LineaBio Inc.

Provide early-stage CGT companies with GMP-compliant iPSC lines

Toronto • Preclinical

Founded in 2023, LineaBio Inc. was spun out of CCRM and OmniaBio to offer customers accelerated access to affordable and GMP-grade iPSC lines. By sharing iPSC sub-clones among multiple companies, LineaBio's model promises to reduce development costs while still allowing for the development of Master Cell Banks and downstream differentiation to produce a final drug product. By leveraging CCRM's expertise and OmniaBio's large-scale manufacturing, LineaBio will ensure access to services for end-to-end manufacturing of cell and gene therapies (CGT) for its customers.

Mediphage Bioceuticals

Next-generation non-viral gene therapy platform

Toronto/Boston • Preclinical

Mediphage has strategically expanded its reach by entering into several agreements and commencing studies with prominent global life science companies, collaborations encompassing areas such as rAAV and mRNA production, gene addition and gene editing. Mediphage is actively engaged in collaborative efforts with both public and private organizations, aiming to apply msDNA to their diverse programs, underscoring Mediphage's dedication to contributing to the development of solutions in the Canadian life science and innovation ecosystem.

Mesentech

Delivery of regenerative molecules to bone

Vancouver • Phase 1

Mesentech dosed its first patients with MES1022, a subcutaneous bone-selective EP4-agonist for bone regeneration. MES1022 is the first *de novo* designed pharmaceutical with tissue-selective distribution to bone to quicken the healing of bone fractures. A second program using tissue-targeting technology is in development for bone cancers such as osteosarcoma or bone metastases. Through mimicking the body's natural processes to stimulate bone repair and healing, MES1022 aims to prevent patients undergoing expensive and invasive revision surgeries.

Morphocell Technologies

Treating acute liver failure with organ replacement therapy using allogeneic stem cell-derived engineered tissues

Montreal • Preclinical

Morphocell is developing an extensive therapeutic platform based on its allogeneic stem cell-derived engineered liver tissues. Morphocell's first therapeutic product, ReLiver®, allows for the allogeneic treatment of acute and acute-on-chronic liver failure. In 2023, Morphocell made significant progress on its way to the clinic, obtained three patents granted in three different territories, hired leading experts in product and preclinical development, doubled its team to 15 full-time employees, and acquired major equipment.

Turn to page 8 to read more about OmniaBio Inc., and page 15 for the Canadian Advanced Therapies Training Institute.

Notch Therapeutics

Universal stem-cell derived T-cell therapies

Toronto • Preclinical

Notch is developing best-in-class iPSC-derived T cells from CD34+ blood progenitor cells in small footprint bioreactors at a scale sufficient to support multiple batches of T-cell doses in clinical trials. Notch is developing a CRISPR MAD7-based gene editing platform for rapid generation of precisely engineered, multi-edited iPSCs as a starting material to manufacture complex iPSC-T cell drug products that can be designed to treat complex tumours. Notch is progressing its iPSC-T cell pipeline toward clinical development and made progress on proof-of-concept product designs, including demonstrating the feasibility of both CAR- and TCR-engineered iPSC-T cells.

panCELLa (subsidiary of Pluristyx)

Gene engineering solutions for safe iPSC therapies

Toronto • Preclinical

panCELLa has merged with Seattle-based Pluristyx, a CDMO provider of iPSC lines, related products and services. The merged company is using the panCELLa "induced Allogeneic Cell Tolerance iPSC (iACT) Stealth Cells" and "FailSafe" technology platforms, to provide GMP-grade edited iPS cell banks and end-to-end solutions for therapeutic development products. The combined company reached a number of strategic milestones in 2023, including the announcement of major partnerships with groups like ARM and CIRM. panCELLa will continue to operate out of Toronto.

CCRM and Medicine by Design enhance their partnership in 2023

CCRM and Medicine by Design have been commercialization partners since Medicine by Design was established in 2015 by substantial funding from the Canada First Research Excellence Fund (CFREF). The launch of Medicine by Design was led by Peter Zandstra, PhD CM, Chief Scientific Officer of CCRM, and a professor at the University of Toronto at the time. At the end of 2023, we expanded our partnership through a strategic alliance.

The alliance is intended to support Medicine by Design's important work beyond the CFREF investment. As announced on December 6, the partnership will build stronger linkages between CCRM and the curated regenerative medicine discovery pipeline that Medicine by Design has built. More broadly, it will leverage both the University of Toronto's and University Health Network's reputation for world-class research, medicine and a combined network of regenerative medicine-focused faculty, clinicians, as well as key opinion leaders, social scientists and other non-STEM investigators.

Both parties are eager to build upon their strengths: bridging high-risk, high-reward research combined with industry expertise, biomanufacturing infrastructure and the clinic. Ultimately, patients will benefit from this enhanced relationship that will help more therapies reach the marketplace sooner.

Summer by Design

Summer by Design, an intensive program for PhD students and postdoctoral fellows from Canadian and international universities, was back in 2023 after being postponed due to the COVID-19 pandemic. A cohort of 30 participants, along with Medicine by Design organizers and presenters from CCRM, the Rotman School of Management and the Stem Cell Network, were very excited to impart knowledge on topics related to regenerative medicine commercialization. From intellectual property to regulatory affairs, clinical translation, biomanufacturing, reimbursement and technology adoption – to name a few topics – the 10-day program kept the participants mentally and physically engaged.

Socializing and networking are also built into the schedule as building an international network is an important feature of Summer by Design – and one that is highly valued. As one participant summed it up: "The friendships I made in two weeks were invaluable."

CCRM and Medicine by Design are excited by the opportunities and synergies our strengthened partnership will produce, and look forward to more announcements in the future.

Michael Sefton, Leah Cowen and Allison Brown of the University of Toronto, and Michael May.

Key Funders

NCE RCE

Federal Economic Development Agency for Southern Ontario

Agence fédérale de développement économique pour le Sud de l'Ontario

Ontario

Founding Institutional Members

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EMPIRICA Therapeutics

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 utilized 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.

Commercializing
Living Therapies

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