# 2022 Annual Report SUSTAINED IMPACT

Making waves in the cell and gene therapy ecosystem



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.

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### **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.

### **Board of Directors**

CHAIR -

Michael Nobrega

IBI Group, Canada

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**Claudia Zylberberg** Akron Biotechnology, USA

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

2022 ANNUAL REPORT

# The CCRM-OmniaBio Team

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## **Channeling momentum:** A roadmap for the next 10 years

Last year's annual report was a celebration of CCRM's first 10 years. It was really quite incredible to reflect on what the team has accomplished in only a decade. (If you missed it, catch up here.) We leveraged the modest infusion of seed funding from the federal Networks of Centres of Excellence (NCE) program. Without this important support, CCRM would not have been able to help our academic partners and portfolio companies attract ~\$1.2 billion, build a team of 220 personnel dedicated to cell and gene therapy commercialization, and construct specialized product development and biomanufacturing process development infrastructure. We are very grateful to the NCE for realizing Canada's potential in regenerative medicine and making funding available to support our ecosystem-building activities. We also want to thank the Government of Canada, through the Federal Economic Development Agency for Southern Ontario, and Next Generation Manufacturing Canada, for project-specific funding support.

What comes next? Some of that was hinted at in last year's annual report Our NCE funding ends in the first quarter of 2023 as we have reached the maximum funding allowable. Having graduated from the NCE program, the organization is continuing its transition to sustainability through our contract services, company exits and investments. Our plan is to accomplish this by growing our manufacturing capabilities, catalyzing access to capital, providing specialized training, and re-investing in the academic pipeline. Moreover, we are starting to prioritize establishing CCRM hubs across the country, and the world, to scale activities and build on the successful model we have created in Ontario.

As you read through the pages of this annual report, you will notice that we are already delivering on our goals:

- Grow manufacturing capability OmniaBio Inc.
- Catalyze access to capital CCRM Enterprises
- Provide specialized training –
  Canadian Advanced Therapies Training Institute (CATTI)
- Re-invest in the academic pipeline The CCRM Foundation
- Establish national and global hubs CCRM Australia

We will look back on OmniaBio as a watershed moment. Similar to the establishment of the Centre for Advanced Therapeutic Cell Technologies – with thanks to Cytiva – and building the Centre for Cell and Vector Production – in partnership with University Health Network – our specialized infrastructure has already had a monumental impact on our operations and ability to influence the sector.

Not only was creating and launching OmniaBio Inc. the result of a tremendous amount of effort from across the organization, but it also required confidence in our vision on the part of our investors. Thank you to Invest Ontario and Medipost for your trust. We will continue to support our subsidiary to help it thrive. We are passionate that commercial-scale biomanufacturing is crucial for Canada to be a player - and ideally a leader - in the flourishing cell and gene therapy industry. Without domestic capability, the companies that are launched in Canada will move to other jurisdictions where manufacturing infrastructure is better developed. We believe sustainability will

happen through the creation of a seamless technology to market pipeline – which we are building – that brings "stickiness," through manufacturing capability, access to capital and talent development, to Canada's ecosystem.

Speaking of supporting the ecosystem, we are pleased to announce that CCRM was selected to work with the National Research Council of Canada to create a not-for-profit to govern the Biologics Manufacturing Centre in Montreal, Quebec. CCRM will provide oversight and strategic direction through its representation on the board of directors of the newly created Biologics Manufacturing Centre Inc.

With OmniaBio underway and being led by an experienced management team, CCRM will focus its attention on more company creation, new services to serve academics and industry, sector-focused investment, philanthropy to support research and education, and working with CellCAN to ensure that CATTI is meeting Canada's biomanufacturing workforce training needs. We also have exciting plans for engaging with our employees and alumni, and thoughts on how to celebrate the achievements of the global cell and gene therapy sector.

We are very excited about what the next decade will bring and look forward to collaborating with government, institutions, associations, partners, and Canada's regenerative medicine community as we strive to generate sustainable health and economic benefits for Canada and beyond. We hope you will join us on this important mission.

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Michael H. May President and CEO



Peter Zandstra C.M. Chief Scientific Officer

# 2022 Highlights







### Peter Zandstra appointed Member of the Order of Canada

In January, we were delighted to learn that Peter Zandstra, CCRM's co-founder and Chief Scientific Officer, was appointed as a Member of the Order of Canada (C.M.) for "his pioneering leadership in the field of stem cell bioengineering and its subsequent innovative health and economic impacts."

### Commercializing Living Therapies with CCRM podcast

CCRM added a podcast to the resources it produces to enable training and education in our field and continue to advance knowledge through thought leadership. The first season is complete, and Season 2 is underway, featuring academic and industry leaders as guests, and timely topics. Listen on your favourite streaming platform or <u>CCRM's website</u>.

## 10-year anniversary of *Signals*

Time flies when you're busy blogging! Since *Signals* launched 10 years ago, we have been consistently producing and sharing compelling content with readers. To mark the anniversary in May, *Signals* published a month's worth of special content. Visit <u>signalsblog.ca</u>!

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### **CELLS I SEE**

Congratulations to Erin Roberts, University of Calgary, Grand Prize winner for "Embracing Differentiation in OurCELLves." This was the entry with the most votes from delegates at the annual Till & McCulloch Meetings.





### CDL's new stream brings together CCRM and four top universities

In June, Creative Destruction Lab (CDL) and CCRM announced their partnership in a new global Advanced Therapies stream to mentor start-up founders ready to bring their technologies to patients. The stream is taking place at four CDL locations – the University of British Columbia, the University of Oxford, the University of Toronto, and the University of Wisconsin-Madison – and a successful launch event took place in Vancouver in October.

### New collaborations anticipated to advance technologies

CCRM publicly announced collaborations with CELL AG TECH (April), Resolution Therapeutics (November) and Avectas (December) to work with our skilled process development and GMP teams to scale up their technologies for manufacturing. From cellular aquaculture to induced pluripotent stem cells, CCRM is enabling technology companies who are ready to move to manufacturing.

## CCRM creates an independent NFP corporation with the NRC

Following a formal application process, CCRM was selected to work with the National Research Council of Canada (NRC) to create a not-for-profit (NFP) to govern the Biologics Manufacturing Centre (BMC) in Montreal. Built to respond to pandemic and other health emergency preparedness needs, support public-interest projects, and contribute to the growth and resilience of Canada's life sciences sector, the BMC is a key element in the Government of Canada's Biomanufacturing and Life Sciences Strategy.

MUSCLE FLORA



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### **CELLS I SEE**

Congratulations to Lupann Rieger, Université de Montréal, winner of the People's Choice for "Muscle Flora." The People's Choice is awarded to the entry with the highest number of likes on Facebook.

# Focused CGT Manufacturing

## **OmniaBio**

- MAR 31

After years of planning, we were very excited to launch OmniaBio Inc., a subsidiary of CCRM that will enable pre-clinical to commercial-scale manufacturing. As a contract development and manufacturing organization (CDMO), it will provide services for cell and gene therapy (CGT) developers around the world. OmniaBio will be the largest CGT focused CDMO in Canada, setting the province of Ontario on a course to become a global hub for CGT manufacturing. OmniaBio is the first project to be funded by the government's Invest Ontario Fund, enabling the construction of its first building at McMaster Innovation Park, at an initial 100,000 ft<sup>2</sup>.

Minister ••• Vic Fedeli

Michael May, CCRM •••

#### . . . . . .

"As the first facility of its kind to provide commercial-scale cell and gene therapy manufacturing in Canada, OmniaBio will support our pipeline of homegrown life sciences companies, while boosting Ontario's presence in the global biotech industry. This is the kind of value-added, strategic investment that our government is proud to champion – creating skilled jobs, advancing our health care and driving innovation."

- Vic Fedeli, Minister of Economic Development, Job Creation and Trade, Government of Ontario

2022 ANNUAL REPORT

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PARK

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Ontario 😚

BUILD

BUILDING

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MAY 31 In May, OmniaBio announced it had executed a strategic agreement with Medipost, a South Korea-based leader in stem cell therapeutics. In total, Medipost has invested \$90 million into OmniaBio. The funding from Medipost has contributed to an overall project worth \$580 million for the operations, construction and fit out of OmniaBio.

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"CCRM has spent a great deal of time looking for the right investment partner for OmniaBio, and we are very pleased to have Medipost on board. Medipost will be an initial anchor and revenue-generating customer of OmniaBio and will also help us develop an international customer base in Asia."

- Mitchel Sivilotti, CEO, OmniaBio

— **SEP** 20

Next Generation Manufacturing Canada (NGen) has made its largest investment to date – \$10.5 million of \$34.8 million – in a project led by OmniaBio. OmniaBio and its partners – ExCellThera, Morphocell Technologies, Aspect Biosystems and the Canadian Advanced Therapies Training Institute – will strengthen Canada's global leadership in life sciences by delivering against three core objectives: building domestic expertise and world-leading manufacturing capabilities; empowering a pipeline of Canadian-based CGT companies; and training Canada's future biomanufacturing workforce.

#### $\bullet \bullet \bullet \bullet \bullet \bullet$

"This new \$34 million project will not only solidify Canada's leadership but also help train more Canadians for these good, well-paying jobs. Projected to create hundreds of new jobs just in the short term, this new project led by OmniaBio and its consortium partners will make a real difference here at home and around the world."

- François-Philippe Champagne, Minister of Innovation, Science and Industry

OCT 6 CC of

CCRM and OmniaBio executives marked the beginning of construction at the new biomanufacturing campus at McMaster Innovation Park (MIP) in Hamilton, Ontario. Investors Medipost and Invest Ontario joined executives from builder Multiplex, CCRM, OmniaBio and MIP to recognize the occasion.

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"CCRM has been looking forward to this day for a long time. If Canada can establish biomanufacturing capabilities for cell and gene therapies, it can be a leader in this thriving and important field of medicine. With today's event, we are putting Ontario on the map as a destination for Canadian and global companies to manufacture their products here and we are inviting others in the supply chain to come to Hamilton."

- Michael May, President and CEO, CCRM

RM specializes in regeneratine, accelerating the translation of translation of the translation of t

Commercializing Living Therapies

> L-R Michael May (CCRM), Mitchel Sivilotti (OmniaBio), Antonio Lee (Medipost)

# CCRM **Enterprises**

Building a continuum of funding vehicles to support development and manufacturing for cell and gene therapy companies.

Commercializing

Living Therapies

In December 2021, CCRM launched CCRM Enterprises, its venture investment arm, to distinguish its for-profit commercial and investment endeavours. Now firmly established, CCRM Enterprises, led by Cynthia Lavoie, President and Chief Investment Officer, catalyzes new company innovation and investment in regenerative medicine by pulling together all the necessary components to vet, de-risk and develop high-potential, early-stage ventures as they scale up along the development and commercialization pathway.

The goal of CCRM Enterprises is to address gaps in the Canadian investment ecosystem. With extensive deal sourcing capability, supported by "wet" diligence through CCRM's technical teams, CCRM Enterprises can then progress these technologies, enabled by CCRM's strengths in manufacturing and technology development. Companies in CCRM Enterprises' portfolio also benefit from access to the Good Manufacturing Practices (GMP) facility at CCRM.

CCRM Enterprises typically invests in companies at the pre-seed and seed stages of financing. CCRM Enterprises works closely with its strong network of co-investors to attract later stages of investments to its portfolio of companies. At the heart of CCRM Enterprises is a financing continuum of fit-for-purpose investment vehicles. enabling investment that is custommatched to the stage and risk profile of the investment target.

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Service for Equity

& Manufacturing

**Co-investor Network** 

Investment areas for targeted investment include. but are not limited to: gene-modified cell therapies (viral and non-viral in vivo cell engineering, third-generation/nextgeneration chimeric antigen receptors with novel targets, natural killer cells, and more); *in vivo* gene therapies (gene therapy in solid tumours and rare disease, novel AAV capsid platforms, gene delivery vehicles, and more); and, editing technologies (third-generation gene editing technologies and epigenome editing technologies).

CCRM Enterprises' direct investment strategy is two-fold: finding strategic alignment with CCRM's process development and GMP-compliant manufacturing capabilities (ExCellThera, panCELLa, Notch Therapeutics, Morphocell Technologies, iVexSol and Exacis Biotherapeutics); and investing in adjacent cell and gene technology areas (as with Endogena, KisoJi, Feldan Therapeutics, Mediphage Bioceuticals, Aspect Biosystems and Mesentech).

On pages 17 to 19, we check in with CCRM Enterprises' portfolio companies to learn about their most recent highlights.





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**IP** Sourcing

**Direct Investments** 

Fit-for-Purpose Ø **Investment Vehicles** 

# The CCRM Foundation

Using philanthropy to promote, advance and support the regenerative medicine ecosystem in Canada.

If you regularly follow announcements from

It is a private foundation that will promote,

medicine ecosystem to advance life-changing

foundations in our field. By identifying gaps in the ecosystem and then funding impactful

philanthropy, The CCRM Foundation will play

an important role in building sustainability

Over the last few years the team at CCRM,

led by Donna Masters, Director of Strategic

Philanthropy, has been working behind

the scenes to build the framework and

infrastructure of The CCRM Foundation

to ensure complete legal compliance and

thoughtful planning. As the needs of the

regenerative medicine ecosystem and

solutions to fill these gaps via strategic

and supporting cell and gene therapies.

CCRM, you may have noticed that we've

advance and support the regenerative

therapies, in collaboration with other

our community outreach evolve, and our Board of Directors identifies innovative mentioned The CCRM Foundation in the past. ways to contribute, our giving priorities and programs will shift to address this proactive thought leadership. We will be nimble and progressive to ensure high impact philanthropic investments. We will always strive to steward donor funds in a transparent and collaborative manner, engaging donors in the philanthropic process to support the ecosystem.

> The CCRM Foundation will also look beyond the traditional regenerative medicine ecosystem to build partnerships with key community organizations that support building systems that increase the accessibility of science-based education and opportunities to underserved children and youth. The CCRM Foundation is committed to adopting and living the values of inclusion, diversity, equity and access.



Claudia Zylberberg, CEO of Akron Biotech, and a member of CCRM's Board of Directors. published an updated version of her children's book, You're *Full of Genes*, and shared proceeds of sales with The CCRM Foundation and two other organizations. We thank Dr. Zylberberg for her efforts to educate the public, beginning with young children, and for her generosity.

### GLOBAL HUBS

# CCRM Australia

Commercializing Living Therapies The CCRM team has bold ambitions, believing that global scaling and international collaboration are key to building the industry around Canadian leadership. That means facilitating the bundling of IP, sharing expertise and leveraging funding across all major markets.

CCRM Australia was launched in 2016. The first hub in a global network of hubs, CCRM Australia was established through a partnership by CCRM with Monash University, the Commonwealth Scientific and Industrial Research Organisation (CSIRO), St Vincent's Health and Cell Therapies Pty Ltd, with additional funding from the Victorian and Australian Governments. It is led by Silvio Tiziani, CEO, and Dr. Chih Wei Teng, COO.

Like CCRM, CCRM Australia accelerates the commercialization of regenerative medicine therapies and related technologies. It does this by providing expertise, funding opportunities and connections between its various networks comprising industry, academics, clinicians and other key stakeholders. CCRM Australia's vision is to "seek to enable, integrate, engage and internationalize the Australian regenerative medicine industry." In December 2021, it achieved an important milestone when each of the founding organizations renewed their financial commitment.

CCRM Australia has a well-established training and education program that includes student internships and opportunities for PhDs and postdocs, webinars for industry professionals, and a partnership with the Canadian Advanced Therapies Training Institute (CATTI). In January 2022, CCRM Australia and CATTI entered into an agreement to advance cell and gene therapy training in Australia. CCRM Australia gains access to CATTI's training programs and the arrangement allows CATTI to access Australian subject matter expertise through the CCRM Australia Partner Network to support the adaptation and further development of CATTI programs for an Australian and global market.

While CCRM Australia continues to grow and advance its offerings, other countries are working to establish their own organizations replicated on CCRM's successful model. The increased international collaboration that we will see in the future will benefit Canadians, patients and the global regenerative medicine community.

You can follow CCRM Australia on Twitter, LinkedIn and through their newsletter (sign up on their website).

### TRAINING AND EDUCATION

# Canadian Advanced Therapies Training Institute (CATTI)

Advanced Therapies GMP Onboarding course graduates from Aspect Biosystems



In July 2021, CCRM and CellCAN, a knowledge mobilization network in cell and gene therapies, launched the Canadian Advanced Therapies Training Institute (CATTI). With over 70 courses available and more than 280 highly qualified professionals (HQPs) already trained in its first year of operation, CATTI is working to close the biomanufacturing training gap that exists in Canada.

The Institute is ensuring Canada's flourishing biomanufacturing industry can keep up with the pace of progress, by offering e-learning and on-site programs that enable its students to meet role-specific competencies for cell and gene therapy (CGT) manufacturing jobs.

CATTI's best-in-class programs enable therapeutics developers to address the global biomanufacturing talent shortage head-on, in a way that doesn't compromise the industry's exacting standards and skills requirements. Programs are carefully reviewed by a committee of CGT subjectmatter experts who ensure programs comply with current Good Manufacturing Practices (GMP) requirements.

Its curriculum has been organized into five themes: Good Manufacturing Practices; Aseptic Cleanroom Systems; Manufacturing (upstream, downstream and fill-finish); Quality Assurance; and, Regulatory Affairs. Once trained, HQPs obtain certification under GMP and are qualified to work under aseptic conditions to manufacture cancer immunotherapies, CGTs, vaccines and other biotherapeutic applications.

CATTI is building an offering that goes beyond the theory, including the hands-on practicum that is crucial for employees to excel in a work environment so they are fully prepared for what they will encounter. Partnering with CCRM and OmniaBio, CATTI is on track to open its first handson containment level 2 training site at the University of Guelph, in the Ontario Veterinary College, in spring 2023. It will provide participants with skills development opportunities in CGT manufacturing, from stem cells and T cells to viral vector production.

CATTI has been recognized as OmniaBio's preferred training provider and, to date, more than 50 employees from CCRM and OmniaBio have already been trained by the Institute.

# Ecosystem Training Events

– TMM workshop Vishan Sivagnanam, CCRM

CCRM is pleased to return to hosting and collaborating with partners on in-person workshops and events geared towards training and knowledge sharing. Here are some of this year's highlights:

This summer, CCRM hosted a commercialization workshop at the **TERMIS 2022 Americas** conference in Toronto. The workshop "From bench to boardroom: Commercializing your RM product or therapy," saw CCRM experts, industry facilitators and keynote speaker Tamer Mohamed, Aspect Biosystems, come together to deliver a varied program that gave a broad overview of what it takes to commercialize a cell or gene therapy. In October we hosted a commercialization workshop in Vancouver at the **Till & McCulloch Meetings** (TMM). Attendees heard about CCRM's unique company creation and incubation programs. The workshop highlighted how CCRM's experts engage with academics to develop promising IP, and how to assess commercial readiness along with key considerations for early commercialization and scaling up. CCRM was also pleased to support TMM by sponsoring the inaugural career fair, impactful patient talks, and an Equity, Diversity and Inclusion lunch.

• TERMIS workshop

Panel at CDL event with CCRM's Cynthia Lavoie, right.

CCRM and **Creative Destruction Lab** (CDL) hosted their first event in October as part of the new global Advanced Therapies stream. The Vancouver event featured keynote talks, a panel, and a dinner. The following day, company representatives pitched their technologies to CDL mentors hoping to be selected to join the stream. The stream is designed to support pre-seed and seed-stage ventures at the forefront of biotech that are keen to bring their innovations to patients. Together with Janssen, Johnson & Johnson Innovation and JLABS Toronto, CCRM hosted a special gathering featuring cell and gene therapy (CGT) thought leaders. Including a company showcase and several panels, the Toronto event, held in November, explored how, as an ecosystem, we can drive the development of CGTs to bring innovations to patients more efficiently. One week later, **3M**, with collaborators CCRM, **Life Sciences Ontario** and **Ontario Bioscience Innovation Organization**, brought together top industry experts to discuss the future of biopharmaceutical processing and biomanufacturing in Canada. The day-long event featured talks including "launching commercial CGT-focused biomanufacturing in Canada" and "from COVID-19 to pandemic preparedness."

## Portfolio Company Highlights

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CLICK THE BOXES TO GET TO THEIR WEBSITES!



Aspect Biosystems Microfluidic 3D bioprinting of human tissues

Vancouver • Preclinical

Aspect Biosystems has expanded the size of its Vancouver headquarters by over 40 per cent and added 35+ new team members, bolstering its therapeutic development and GMP manufacturing. Eight new board members and scientific advisors also joined Aspect, bringing significant cell therapy expertise and leadership. 📩 endogena

**Endogena Therapeutics** Regenerative molecules for retinal degenerative disease

#### Toronto/Basel • Preclinical

Endogena Therapeutics won the prestigious Retina Innovation Award and announced its transition into the clinical stage with its novel regenerative treatment for blindness. It treated its first patient in a Phase 1/2a clinical study, testing lead product EA-2353, and tested drug candidate EA-2351 in two animal models with promising effects. Endogena is working on a project to treat idiopathic pulmonary fibrosis, allowing it to diversify its product pipeline. **EXACIS** BIOTHERAPEUTICS

#### Exacis

in the last year.

Messenger RNA reprogramming platform for engineered T and NK cells *Boston* 

Exacis has raised \$3M to advance its process development activities, *in vitro* functional testing, and to further develop its freeze/ thaw processes of iPSCs and key intermediary cells. Exacis signed an option agreement with Eterna Therapeutics to license up to four off-the-shelf, iPSC-derived NK cell and T-cell cancer treatments.



**ExCellThera** Optimized cord blood grafts for improved HSCT

Montreal • Phase 2

*Through CCRM Enterprises, CCRM provides tailored support to advance the translation of promising discoveries from lab* 

14<sup>\*</sup> companies that have gone on to raise almost \$1B. Here are some notable achievements from our portfolio companies

to market, and has supported the launch and growth of

ExCellThera's ECT-001 Cell Therapy has been administered to over 100 patients in Phase 1/2 trials across multiple hematological indications. Top-line data across all trials are expected in the first half of 2023 and ExCellThera is looking forward to working with regulatory authorities to secure market access.



#### **Feldan Therapeutics**

Therapeutics using non-viral protein transport shuttle

Quebec City • Discovery

Feldan closed a USD\$2M bridge financing, allowing the completion of a Phase 1/2 clinical trial for Gorlin syndrome. Feldan started a collaboration with a U.S. biopharma company in 2022, and received \$2M in grants. Feldan's scientific team continues to optimize the use of its flagship Shuttle platform in lungs, enabling them to test instillation in non-human primates. iVe×Sol

**iVexSol** Manufacturer of stable lentiviral vector cell line

Toronto/Boston • Discovery

In September 2022, iVexSol and Cellares Corporation announced a strategic partnership to advance viral vector manufacturing. iVexSol will provide viral vector production supporting the development of Cellares' Cell Shuttle program. iVexSol has increased hiring and is building its Board of Directors and Scientific Advisory Board.



**KisoJi Biotechnology** Single domain antibodies against

hard to drug targets Montreal • Preclinical

Kisoli has developed a preclinical pipeline that includes a naked TROP2 antibody with significant single agent anti-tumor effects. To date, the lead TROP2 antibody has shown robust therapeutic efficacy; early pre-clinical data demonstrate shrinkage in tumour volume by 71 per cent in a SCLC xenograft model. In Fall 2022, Kisoli is closing a large Series B financing.

## MEDIPHAGE

### **Mediphage Bioceuticals**

Next-generation non-viral gene therapy platform

Toronto/Boston • Preclinical

Mediphage's preliminary studies evaluating an msDNA construct harbouring a therapeutic gene for Dravet syndrome demonstrated promising results in Dravet mice models, laying the foundation for upcoming preclinical studies. Another study evaluating an msDNA construct encoded with a therapeutic gene for Hemophilia A showed strong Factor VIII activity in Hemophilic mice models. Mediphage recently signed an Option to License agreement with a major pharma company for its msDNA technology.

### O-O Mesentech

### Mesentech

Delivery of regenerative molecules to bone

Vancouver • Preclinical

Between 2019 and 2022, Mesentech demonstrated safe and effective bone regeneration in mice and NIP models and, in April 2022, announced the closing of a seed funding round. Mesentech is developing an EP4 agonist that promotes bone formation to tackle non-union bone fractures and other bone degenerating diseases. In 2023, it will commence its Phase 1 trial in the Netherlands.



**MorphoCell** Encapsulated hepatocytes for Acute Liver Failure

Montreal • Preclinical

Morphocell significantly de-risked their technology ReLiver®, collecting preclinical efficacy and safety data, and addressing key hurdles in process development. Over the last year, Morphocell more than doubled its team, established key strategic partnerships, relocated into new state-of-the-art labs and headquarters, and kicked-off fundraising for Series A financing.



Notch Therapeutics Universal stem-cell-derived T-cell therapies

Toronto • Preclinical

Notch has built the capabilities of an end-to-end iPSC therapeutics company with more than 90 employees at three North American sites, since launching in 2018. In 2021, Notch closed USD\$85M Series A financing and, in 2022, the company achieved critical proof of concept and scalability milestones.



### panCELLa

Gene engineering solutions for safer iPSC therapies

Toronto • Discovery

Between 2019 and 2022, panCELLa signed several co-development agreements and, in October 2022, Pluristyx, PanCELLa and Implant Therapeutics announced a merger, combining complementary portfolios to enable increased access to a wide range of iPSC-related products and services.

# **Extending Frontiers in Regenerative Medicine**



Medicine by Design is a convergence hub for scientists, engineers and clinicians to conceive and translate regenerative medicine discoveries into new therapies to transform human health. Medicine by Design received an unprecedented investment of \$114 million from the Canada First Research Excellence Fund. Ever since, it has been pushing the frontiers of regenerative medicine at the University of Toronto, and its affiliated hospitals, through transformative investment into interdisciplinary research projects and the recruitment of 17 world-class faculty members. Medicine by Design and CCRM have been commercialization partners since its launch in 2015. Through their strategic partnership, Medicine by Design is integrating discovery and early-stage translation in regenerative medicine with sector-specific expertise in commercialization, biomanufacturing and clinical implementation.

In 2022, Medicine by Design announced more recipients of its Pivotal Experiment Fund, designed to bridge the "valley of death" – a critical gap between funding from the government for discovery research as well as funding from capital investors or industry partners. With a total of \$2.5 million distributed since 2021, the Pivotal Experiment Fund now supports ten innovations that range from an efficient mRNA delivery platform to a hydrogel that delivers a cell therapy into the eye, to a gene therapy for reprogramming brain cells.

Medicine by Design's Entrepreneur-in-Residence (EIR) program also expanded in 2022, adding two seasoned advisors to work alongside the EIR. This program provides researchers with mentorship and an entrepreneurial perspective on product and/or venture concepts that are emerging from Medicine by Design funded discoveries. The EIR also builds connections between the cell and gene therapy industry and venture capital communities to researchers in the Medicine by Design community.

Through a new strategic plan published in 2022, Medicine by Design also affirmed its commitments to advancing transformative research and early-stage translation across the Toronto Academic Health Sciences Network enabling the commercialization of accessible, living therapies at scale, and preparing health systems, clinics and communities to implement innovations. As Medicine by Design moves toward implementing these goals, it is working with CCRM and other ecosystem partners to address the scientific, clinical, social and economic complexities of creating novel living therapies.

### **Key Funders**



Federal Economic Development Agency for Southern Ontario Agence fédérale de développement

économique pour le Sud de l'Ontario



### Founding Institutional Members



### **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 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

### **CCRM**

MaRS Centre, West Tower 661 University Avenue, Suite 1002 Toronto, ON, M5G 1M1 416-978-3751

www.ccrm.ca



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