

Quick Facts on Regenerative Medicine and CCRM

Regenerative medicine, including cell and gene therapies, harnesses the power of (stem) cells, biomaterials, molecules and genetic modification to repair, regenerate or replace diseased cells, tissues and organs. This approach is disrupting the traditional biotechnology and pharmaceutical industries with the promise of revolutionary new cures for devastating conditions such as heart disease, diabetes and cancer.

The Industry

- The global market for regenerative medicine was valued at US\$13.3B in 2019 and forecasted to grow to US\$73.4B by 2027, at a CAGR of 23.7 per cent during the forecast period.¹
- Globally, regenerative medicine companies raised more than US\$9.8B in 2019, which is the second-strongest year in financings to-date.²
- Approximately 100,000 patients will be treated with CAR-T immunotherapies by 2021.³
- At the close of 2019, there were 1,066 total clinical trials in regenerative medicine and advanced therapies products approved by international regulatory agencies, including 94 in Phase III development.²
- The majority of clinical trials were in oncology (62%), while musculoskeletal clinical trials came in second (5.6%).²
- Canada ranks second in cost competitiveness for biomedical R&D compared to other industrialized nations.⁴
- North America holds 39 per cent of the global regenerative medicine market.⁵

There is increased intensity of regenerative medicine industry activity. Recently, we've seen the following:

- Select recent approvals of new cell and gene therapies include Novartis' Luxturna (October 2020) and Kymriah (September 2018) by Health Canada, Kite Pharma's Tecartus (July 2020) and Yescarta (March 2018), and Novartis' Zolgensma (May 2019) by the U.S. Food and Drug Administration, and Bluebird Bio's Zynteglo (March 2019) by the European Medicines Association.
- In March 2020, Editas Medicine and Allergan announced the first use of *in vivo* CRISPR gene editing in a patient for the treatment of an inherited form of blindness.
- In December 2019, Astellas paid US\$3B for Audentes Therapeutics, expanding its gene therapy offering.
- In November 2019, Allogene Therapeutics and Canada's Notch Therapeutics announced a collaboration to research and develop induced pluripotent stem cell-derived allogeneic therapies for hematologic cancer indications. Notch Therapeutics was launched in 2019 as the first "graduate" of CCRM's incubation program.
- The US\$1B acquisition of BlueRock Therapeutics by Bayer AG, in August 2019, demonstrates Toronto's ability to research, manufacture and commercialize a breakthrough therapy. BlueRock Therapeutics' cardiac program leverages intellectual property from Dr. Gordon Keller at Toronto's University Health Network, and CCRM supports the manufacturing platform.
- In July 2018, AVROBIO raised more than US\$100M in an initial public offering on the NASDAQ.

* Dollar amounts are CAD unless otherwise stated.

1 Regenerative Medicine Market by Type [Cell-Based Immunotherapy & Cell Therapy (Allogeneic & Autologous Products), Tissue Engineering, Gene Therapy], Applications (Wounds & Dermal, Musculoskeletal, Oncology), Region - Global Forecast to 2024. [marketsandmarkets.com](https://www.marketsandmarkets.com). Website, March 2020

2 Alliance for Regenerative Medicine 2019 Annual Report

3 Kitamura, Makiko. GE Sees Cell Therapy Industry's Sales at \$10 Billion by 2021. Website, 2015

4 KPMG Competitive Alternatives, 2016

5 Global Regenerative Medicines Market – Analysis and Forecast (2017-2025) (Focus on Therapy, Applications, Market Share Analysis, 22 Country Analysis, and Competitive Landscape). [reportlinker.com](https://www.reportlinker.com). Website, March 2020

Media Contact:

Stacey Johnson, Director of Communications & Marketing

E: stacey.johnson@ccrm.ca; T: 416-946-8869

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CCRM's Commercialization and Scientific Strengths

Commercialization is the process of bringing a new product to market. CCRM specializes in developing and commercializing regenerative medicines, which include cell and gene therapies, and the associated enabling processes and technologies.

CDMO Selected Highlights

- In 2016, Cytiva (formerly GE Healthcare Life Sciences) and the Canadian government each contributed \$20M for CCRM to build a fully-resourced, 10,000 ft² (~930 m²) facility to advance manufacturing and process development activities for therapy developers. Called the Centre for Advanced Therapeutic Cell Technologies (CATCT), it assists developers with the establishment and optimization of industrial-scale manufacturing workflows, as well as by developing new technologies to help solve emerging technical challenges. Cytiva renewed its commitment to CATCT in 2019.
- In 2019, CCRM's cell therapy manufacturing capabilities expanded with the opening of a 20,000 ft² (~1,300 m²) Good Manufacturing Practices (GMP) facility, called the Centre for Cell and Vector Production (CCVP). CCVP provides clean rooms, quality assurance and quality control processes, and highly-qualified personnel to companies and academics needing to manufacture cells and viral vectors to perform Phase I and II clinical trials.
- In 2017, CCRM's technology development team produced 35 billion pluripotent stem cells in an 8 L bioreactor culture and 8 billion cardiomyocytes, and scaled lentiviral producer cells up to a 50 L stirred tank reactor.

Incubating Companies

- CCRM supports Canadian start-ups with its business development efforts. As of May 30, 2020, CCRM had invested more than \$3.4M into its 11 portfolio companies. Over 400 opportunities have been assessed, and 25 technology projects are ongoing.

Investing

- CCRM is establishing investment vehicles with strategic partners to finance the commercialization of regenerative medicine-based technologies and cell and gene therapies.

Our Network

- The University of Toronto, one of CCRM's Founding Institutional Members, and Mount Sinai Hospital, an Associate Institution, rank second and fourth, respectively, in the world in terms of scientific stem cell publications.⁶
- CCRM has built an industry network of more than 100 companies and launched more than 10 co-development projects with industry partners to commercialize regenerative medicine-based technologies and cell therapies.
- CCRM is establishing global hubs to support the commercialization of IP from advanced therapies in locations with opportunities to bolster existing strength in the field. CCRM Australia has already launched.
- CCRM is the commercialization partner for OIRM, a network of over 175 research programs that was awarded \$25M from the Ontario government. We're also the commercialization partner of Medicine by Design, a centre at the University of Toronto that is designing and manufacturing molecules, cells, tissues and organs that can be used to treat degenerative diseases. In 2015, it was awarded \$114M from the Government of Canada.

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⁶ Translational Regenerative Medicine: World Market Prospects 2014-2024

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