Quick Facts on Regenerative Medicine and CCRM

Regenerative Medicine Industry Overview

Regenerative medicine, including cell and gene therapies (CGTs), 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.

Industry Snapshot

- The global regenerative medicine market was valued at US$55.03B in 2022 and is expected to expand at a compound annual growth rate of 15.7 per cent from 2023 to 2030.[1]
- US$12.6B was raised in 2022, which the Alliance for Regenerative Medicine (ARM) calls "a return to normal" given the macroeconomic environment. This figure represents a 44 per cent *decrease* from 2021's US$22.7B, and it is lower than 2020's US$19.9B. However, it is much closer to 2019, which sat at US$9.9B, as well as the years prior.[2]
- In 2022, there were 1,457 cell and gene therapy developers worldwide, which is an 11 per cent increase from 2021. There were 686 companies in North America, 482 in the Asia Pacific region, 244 in Europe, and 35 elsewhere.[2]
- As of April 2023, there were 2,354 active clinical trials in regenerative medicine globally. Of these, 1,843 were in cell therapy (including CAR T-cell therapy and immunotherapy) and 485 were in gene therapy. The major therapeutic areas are cancer (1,615 trials), musculoskeletal diseases (121), blood disorders (89) and cardiovascular diseases (80).[3]
- It is predicted up to 350,000 patients in the U.S. alone will be treated with CGTs by 2030.[4]
- Canada ranks second in cost competitiveness for biomedical R&D compared to other industrialized nations.[5]

Key Milestones from the Past Five Years

- Some recent approvals of CGTs and indications include:
  - Health Canada: Kite's Tecartus for mantel cell lymphoma (approved in October 2021), Celgene's Abecma for multiple myeloma (May 2021), and Kite's Tecartus for r/r mantle cell lymphoma (October 2021).
  - U.S. Food and Drug Administration: Novartis' Zolgensma for spinal muscular atrophy (February 2023), Kite's Yescarta for B-cell lymphoma (November 2022), bluebird bio's Zynteglo for ß-thalassemia (September 2022), and Novartis' Kymriah for r/r follicular lymphoma (July 2022).
  - European Commission: Atara's Ebvallo for lymphoproliferative disease (December 2022), BioMarin's Roctavin for hemophilia A (August 2022), and Orchard's Libmeldy for metachromatic leukodystrophy (December 2020).
- In February 2021, Canada's Notch Therapeutics announced the closing of an oversubscribed US$85M in Series A financing. 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 CRISPR/Cas9 genetic editing tool made headlines. In 2022, as a U.S. patent court ruled in favour of the Broad Institute, MIT and Harvard as CRISPR/Cas 9's inventors, refusing the claims of the University of California, the University of Vienna and Emmanuelle Charpentier. Notably, in 2020, Emmanuelle Charpentier and Jennifer Doudna, both of the latter party, were awarded the Nobel Prize in Chemistry for discovering the tool.
- The 2019 US$1B acquisition of BlueRock Therapeutics by Bayer AG demonstrates Toronto's ability to research, manufacture and commercialize a breakthrough therapy. Its cardiac program leverages intellectual property from Dr. Gordon Keller at Toronto's University Health Network. CCRM supports the manufacturing platform.
- In July 2018, AVROBIO raised more than US$100M in an initial public offering on the NASDAQ. AVROBIO was co-founded by CCRM and has exited from CCRM's portfolio of companies.

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CCRM Commercializing Living Therapies

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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 medicine-based technologies and cell and gene therapies (CGTs), and the associated enabling processes.

CGT Manufacturing

- In 2022, OmniaBio, a subsidiary of CCRM, launched. OmniaBio's facility is Canada's first and largest commercial-scale contract development and manufacturing organization (CDMO) dedicated to CGTs. Located at McMaster Innovation Park in Hamilton, Ontario, OmniaBio will anchor a biomanufacturing centre of excellence and will open in three phases between 2024 and 2026, at a planned 400,000 ft^2 (~37,000 m^2).
- In 2020, CCRM's technology development team reached a significant milestone by producing more than 10 billion pluripotent stem cells in a 10 L single-use stirred-tank bioreactor platform, as reported on in a published paper.
- In 2019, CCRM's cell therapy manufacturing capabilities expanded with the opening of a 20,000 ft^2 (~1,300 m^2) Good Manufacturing Practices facility, called the Centre for Cell and Vector Production (CCVP). CCVP is a contract manufacturing facility that produces clinical-grade cells and viral vectors for Phase 1 and 2 clinical trials for companies and academics developing therapies for patients.
- In 2017, CCRM's technology development team scaled lentiviral producer cells up to a 50 L stirred tank reactor.
- In 2016, Cytiva and the Canadian government each contributed $20M for CCRM to build a fully-resourced, 10,000 ft^2 (~930 m^2) 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 announced its renewed commitment to CATCT in 2020, bringing its total investment in the centre to $55M.

Incubating Companies

- CCRM supports Canadian start-ups with its business development efforts. CCRM's investments (cash and in-kind) have been leveraged significantly, resulting in additional funding and financing totaling almost $1B.
- CCRM assesses over 100 new technologies annually and advances three-to-five company concepts through incubation each year.

Investing

- In December 2021, CCRM launched CCRM Enterprises Inc. to distinguish its not-for-profit operations from its for-profit commercial and investment endeavours. This new entity invests in promising startups, early-stage regenerative medicine-based technologies, and CGT companies.
- By building fit-for-purpose investment vehicles, CCRM Enterprises custom-matches investment to the stage and risk profile of the investment target. It vets, de-risks and develops high potential, early-stage ventures as they scale up along the development and commercialization pathway. It plans to create two-to-three new investments per year through company creation, support for CDMO work, or direct investments.
- As of January 2023, through CCRM Enterprises, CCRM has supported the launch and growth of 14 companies that have gone on to raise almost $1B. CCRM has exited from two companies: AVROBIO and Empirica.

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CCRM's 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.
- We have launched many co-development projects with industry partners to commercialize regenerative medicine-based technologies and cell therapies, including iPSCs, lentiviral vectors, adeno-associated virus, and CAR T cells.
- 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 of Medicine by Design, a strategic hub where scientists, engineers and clinicians converge to conceive and translate regenerative medicine approaches to transforming human health. In 2015, it was awarded $114M from the Government of Canada.

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* Dollar amounts are CAD unless otherwise stated.

1 Regenerative Medicine Market Size, Share & Trends Analysis Report By Product (Cell-based Immunotherapies, Gene Therapies), By Therapeutic Category, By Region, And Segment Forecasts, 2023 - 2030. grandviewresearch.com. Website, April 2023.

2 Alliance for Regenerative Medicine 2023 State of the Industry Briefing.

3 American Society for Cell and Gene Therapy (ASCGT) Clinical Trials Database. Website, April 2023.

4 Quinn *et al.* (2019). Estimating the Clinical Pipeline of Cell and Gene Therapies and Their Potential Economic Impact on the US Healthcare System. *Value in Health Journal, 22*(6). 621-626. https://doi.org/10.1016/j.jval.2019.03.014

5 KPMG Competitive Alternatives, 2016

6 Translational Regenerative Medicine: World Market Prospects 2014-2024

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