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 market for regenerative medicine was valued at US$30.44 billion in 2023 and is projected to grow to US$168 billion by 2034.[1]
- US$11.7B was raised in 2023, which the Alliance for Regenerative Medicine (ARM) calls "a strong foundation for the sector." As of April 30, 2024, ARM reports that US$7.1B has been raised in Q1 2024.[2]
- As of April 2024, there were 2,848 CGT developers worldwide, which is an 11 per cent increase from Q4 2023. There are 1,222 in North America, 972 in the Asia Pacific region, 561 in Europe, and 93 elsewhere.[3]
- As of April 2024, there were 1,751 active clinical trials in regenerative medicine globally. Of these, 442 were in cell therapy, 586 were in gene therapy, and 757 were in cell-based immuno-oncology. Five per cent are in Phase III clinical trials. Thirty-five per cent are in rare conditions, and 58 per cent are in oncology.[4]
- It is predicted up to 350,000 patients in the U.S. alone will be treated with CGTs by 2030.[5]
- Canada ranks second in cost competitiveness for biomedical R&D compared to other industrialized nations.[6]

Recent Milestones

- To date, 11 CGTs have been approved in Canada, and 34 have been approved globally.
- Some recent CGT approvals include:
  - Health Canada: Bristol Myers Squibb's Breyanzi for r/r large B-cell lymphoma (Mar. 2024), Pfizer's Beqvex (Dec. 2023) and CSL's Hemgenix (Oct. 2023) for hemophilia B, and Janssen's Carvykti for multiple myeloma (Feb. 2023).
  - U.S. Food and Drug Administration: Celgene's Abecma for r/r large B-cell lymphoma (Apr. 2024), Pfizer's Beqvez (Apr. 2024), and Orchard's Lenmeldy for metachromatic leukodystrophy (Mar. 2024). Two sickle cell anemia treatments, CRISPR Therapeutic's Casgevy and bluebird's Lyfgenia, both made headlines, and *SNL* sketches, upon approval in Dec. 2023.
  - For approvals from other global countries/regions, please refer to the American Society of Gene and Cell Therapy (ASGCT) and Citeline's quarterly industry landscape report.
- In 2023, it was reported that CAR T-cell therapy, a treatment for cancer, could cause lymphoma. In June 2024, a large Stanford Medicine study reported that the risk of secondary cancers after CAR-T cell therapy is low.
- 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 Notch announced a collaboration to research and develop induced pluripotent stem cell-derived allogeneic therapies for hematologic cancer indications. Notch launched in 2019 as the first "graduate" of CCRM's incubation program.
- 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 supported the manufacturing platform.
- In 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. It merged with Tectonic Therapeutic in January 2024.

Media Contact:
Stacey Johnson, Vice President, Communications and Marketing
E: stacey.johnson@ccrm.ca; T: 647-309-1830

CCRM Commercializing Living Therapies

June 2024

Quick Facts on Regenerative Medicine and CCRM

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

- LineaBio Inc. is a CCRM subsidiary that launched in 2024. LineaBio provides industry with high-quality, off-the-shelf induced pluripotent stem cell (iPSC) lines, manufactured under Good Manufacturing Practices (GMP). Its non-exclusive licensing model enables a reduction of development costs and time to the clinic, benefitting both developers and patients.
- OmniaBio Inc., a subsidiary of CCRM, launched in 2022. 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) GMP 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, and by developing new technologies to solve emerging technical challenges. Cytiva renewed its commitment to CATCT in 2020, bringing its total investment 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 over $1B.
- Each year, CCRM assesses over 100 technologies and advances three to five company concepts through incubation.
- In 2024, CCRM launched Venture by Design. This workflow enables market-driven ideation around regenerative medicine research and technologies, as well as the application of CCRM's commercialization and company creation expertise, to create new ventures.

Investing

- In 2021, CCRM launched CCRM Enterprises Inc., which invests in promising start-ups, early-stage regenerative medicine-based technologies and CGT companies.
- Through CCRM Enterprises, CCRM has supported the launch and growth of 17 companies that have gone on to raise over $1B. CCRM has exited from two companies: AVROBIO and Empirica.
- 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

Media Contact:
Stacey Johnson, Vice President, Communications and Marketing
E: stacey.johnson@ccrm.ca; T: 647-309-1830

CCRM Commercializing Living Therapies

June 2024

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.

Training

- Industry growth will require more qualified, skilled personnel to manufacture and produce CGTs following GMP. In 2021, CCRM and CellCAN launched the Canadian Advanced Therapies Training Institute (CATTI). CATTI is growing biomanufacturing capabilities by offering specialized training to accelerate the workforce's market readiness.

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.[7]
- CCRM has built an industry network of more than 100 companies.
- CCRM has 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 incorporated in 2022, and Sweden-based CCRM Nordic launched in 2023.
- In December 2023, CCRM announced a strategic alliance with Medicine by Design, a strategic hub where scientists, engineers and clinicians converge to conceive and translate regenerative medicine approaches to transforming human health.

---

* Dollar amounts are CAD unless otherwise stated.

1 Global Regenerative Medicine Market Analysis & Forecast to 2024-2034: Market By Product; By Application; By End-user; and By Region. researchandmarkets.com. Website, June 2024.
2 Alliance for Regenerative Medicine Investment Data. April 2024.
3 Alliance for Regenerative Medicine Therapeutic Developers Data. April 2024.
4 Alliance for Regenerative Medicine Clinical Trials Data. April 2024.
5 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
6 KPMG Competitive Alternatives, 2016
7 Translational Regenerative Medicine: World Market Prospects 2014-2024

Media Contact:
Stacey Johnson, Vice President, Communications and Marketing
E: stacey.johnson@ccrm.ca; T: 647-309-1830

June 2024

CCRM Commercializing Living Therapies