

## Quick Facts on Regenerative Medicine & CCRM

Regenerative medicine, including cell and gene therapy, 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 and costly conditions such as heart disease, diabetes and cancer.

### The Industry

- The current global market for regenerative medicine is USD\$36B and forecasted to grow to reach USD\$49.41B by 2021.<sup>1</sup>
- The global market for stem cell therapies was USD\$6.87B in 2016, and the industry is expected to grow to USD\$15.63B by 2025 with an annual growth rate of 9.2 per cent.<sup>2</sup>
- Globally, regenerative medicine companies raised more than USD\$3.8B in the first quarter of 2018, a 135 per cent year-over-year increase from the first quarter of 2017.<sup>3</sup>
- Approximately 100,000 patients will be treated with CAR-T immunotherapies by 2021.<sup>4</sup>
- There were 959 clinical trials in cell, gene and tissue therapy underway worldwide at the close of the first quarter of 2018, with more than 53 per cent of those in oncology and nearly 10 per cent in cardiovascular disorders.<sup>5</sup>
- At the close of 2017, there were 974 total clinical trials in regenerative medicine and advanced therapies products approved by international regulatory agencies, including 82 in Phase III development.<sup>6</sup>
- Canada ranks second in cost competitiveness for biomedical R&D compared to other industrialized nations.<sup>7</sup>

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

- Three cell therapies and one gene therapy were approved globally, including:
  - Novartis' Kymriah
  - Gilead/Kite's Yescarta
  - Spark Therapeutics' Luxturna
  - TiGenix's Alofisel
- Cellectis raised USD\$164M to further continue clinical development of an "off-the-shelf" CAR-T cell therapy in April 2018.
- Allogene launched with USD\$300M Series A financing as of April 2018.
- AVROBIO raised USD\$60M Series B financing in February 2018.
- Kite Pharma announced that it will use Sangamo Therapeutics' zinc-finger nuclease technology to develop allogeneic CAR-T therapies.
- The \$225M investment in BlueRock Therapeutics demonstrated Toronto's ability to research, manufacture and commercialize a breakthrough therapy.
- The rapid expansion of the stem cell and gene therapy industry in the U.S., Japan, Germany and the U.K. are the key drivers for growth of regenerative medicine markets.
- North America currently holds 39 per cent of the global regenerative medicine market.

<sup>1</sup> Regenerative Medicines Market by Therapy (Cell Therapy, Gene Therapy, Immunotherapy, Tissue Engineering), Product (Cell-based, Acellular), Application (Orthopaedic & Musculoskeletal Spine, Dermatology, Cardiovascular, Central Nervous System), Region - Global Forecast to 2021. marketsandmarkets.com. Website, July 2016

<sup>2</sup> Grandview Research Stem Cells Market Analysis Report, June 2017

<sup>3</sup> Alliance for Regenerative Medicine Q1 2018 Data Report

<sup>4</sup> Kitamura, Makiko. GE Sees Cell Therapy Industry's Sales at \$10 Billion by 2021. Website, 2015

<sup>5</sup> Alliance for Regenerative Medicine Q1 2018 Data Report

<sup>6</sup> Alliance for Regenerative Medicine. Website, 2018

<sup>7</sup> KPMG Competitive Alternatives, 2016

## Quick Facts on Commercialization & CCRM

Commercialization is the process of bringing a new product to market. CCRM specializes in developing and commercializing cell and gene therapies and regenerative medicine technologies.

### CCRM's Commercialization and Scientific Strengths

- CCRM's cell therapy manufacturing capabilities will expand with the construction of a 20,000 ft<sup>2</sup> (~1,900 m<sup>2</sup>) Good Manufacturing Practices (GMP) facility, called the Centre for Cell and Vector Production (CCVP), operational in October 2018. It will provide clean rooms, quality assurance and control processes, and highly-qualified personnel, and will be an optimal setting for companies, researchers and non-profits to perform Phase I and II clinical trials.
- In 2016, GE Healthcare and the Canadian government each contributed \$20M (total \$40M) for CCRM to build a fully-resourced, 10,000 ft<sup>2</sup> (930m<sup>2</sup>) facility to advance technologies and process development activities for therapy developers, called the Centre for Advanced Therapeutic Cell Technologies (CATCT). CATCT provides industry with process development facilities and expertise. It assists with the establishment and optimization of industrial-scale manufacturing workflows, as well as by developing new technologies to help solve emerging technical challenges in those same workflows.
- With our customer-focused approach personalized to each client, we provide services tailored to timelines, financing, ambitions and needs. CCRM provides both scientific and business services for emerging and established partners in the regenerative medicine field.
- 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. CCRM delivered over \$2M in contract services.
- CCRM supports Canadian start-ups with its business development efforts. As of the end of 2017, CCRM had invested more than \$1.8M into its portfolio companies. Eighty disclosures were received and reviewed, and 17 regulatory projects were executed for external and internal clients. In 2015, CCRM launched its first company, ExCellThera, in partnership with IRICoR.
- CCRM is creating an investment fund to finance the commercialization of regenerative medicine and cell therapies.
- CCRM has built an industry consortium of more than 45 companies and launched more than 10 co-development projects with industry partners to commercialize regenerative medicine and cell therapy technologies.
- CCRM is the commercialization partner for the Ontario Institute for Regenerative Medicine (OIRM), a network of over 175 research programs<sup>1</sup> that was awarded \$25M from the Ontario government.
- CCRM is also the commercialization partner of Medicine by Design, a regenerative medicine centre at the University of Toronto that will design and manufacture molecules, cells, tissues and organs that can be used to treat degenerative diseases. It was awarded \$114M from the Government of Canada.
- CCRM is establishing global hubs to support the commercialization of IP from regenerative medicine technologies and cell therapies in locations where there is an opportunity to bolster existing strength in the field. In addition to CCRM Australia, planning is underway for CCRM hubs in countries in Europe, Israel, Japan and South Korea.

### CCRM's Network

- The University of Toronto and Mount Sinai Hospital rank second and fourth, respectively, in the world in terms of scientific stem cell publications.<sup>2</sup>
- Greg Bonfiglio, Chair of CCRM's Board of Directors, ranks 14<sup>th</sup> on California Institute for Regenerative Medicine's list of the top 50 most influential people in the global stem cell and cell therapy field.<sup>3</sup>

For more information on CCRM, please contact [stacey.johnson@ccrm.ca](mailto:stacey.johnson@ccrm.ca) or call 647.309.1830. Visit us at [ccrm.ca](http://ccrm.ca).

\* *Dollar amounts are CAD unless otherwise stated.*

<sup>1</sup> Ontario Institute for Regenerative Medicine, June 2016  
<sup>2</sup> Translational Regenerative Medicine: World Market Prospects 2014-2024  
<sup>3</sup> California Institute for Regenerative Medicine, 2013