



EXCELLTHERA

ExCellThera Announces Initiation of Clinical Trial for Cancer Patients
Phase I/II Trial of ECT-100 to begin in early 2016 at multiple Canadian sites

December 21, 2015 (Montreal, QC and Toronto, ON) – ExCellThera is pleased to announce that Health Canada has approved the initiation of a Phase I/II clinical trial of its lead product, ECT-100, for the expansion of hematopoietic stem cells (HSCs). The trial, set to begin in early 2016, will enroll up to 25 patients who require stem cell transplantation for the treatment of acute myeloid leukemia (AML) and other malignant blood disorders, but who lack a suitable donor. The trial will take place at multiple Canadian transplant centres. The lead clinical site will be Maisonneuve-Rosemont Hospital in Montreal.

ECT-100 is a combination of a novel small molecule, UM171, and novel bioreactor technology that targets the expansion of long-term – describing their regenerative capacity – HSCs. ECT-100 expands blood progenitors (CD34+ cells) more than 100-fold and enables the significant expansion of primitive (undifferentiated) HSCs, which is anticipated to provide the robust long-term reconstitution of the blood forming system from small samples of HSCs.

“Health Canada’s approval of ExCellThera’s first clinical trial is an important milestone for the company,” commented Steven J. Klein, CEO of ExCellThera. “ECT-100 will represent a new therapeutic option for patients requiring stem cell transplantation and who lack a traditional donor. We look forward to obtaining initial clinical data for ECT-100 in 2016.”

Approximately 60,000 people in Canada and the United States have been diagnosed with leukemia this year. The success rate of stem cell transplantation is strongly correlated to the number of stem cells given to the patient, making ExCellThera’s stem cell expansion platform a valuable breakthrough for clinical application.

About ExCellThera

ExCellThera is a clinical stage biotechnology company that is focused on developing robust and cost effective ways of growing blood stem cells for therapeutic use. ExCellThera is a spin-off company launched by the Institute for Research in Immunology and Cancer – Commercialization of Research (IRICoR), and the Centre for Commercialization of Regenerative Medicine (CCRM). It is based on novel proprietary intellectual property related to the expansion of stem cells developed by Dr. Guy Sauvageau (Université de Montréal’s Institute for Research in Immunology and Cancer (IRIC)) and Dr. Peter Zandstra (University of Toronto’s Institute of Biomaterials and Biomedical Engineering). The company launched in June 2015 and is developing a range of products, including gene therapy applications, based on its stem cell expansion technology.

About the Institute for Research in Immunology and Cancer — Commercialization of Research

The Institute for Research in Immunology and Cancer — Commercialization of Research (IRICoR) is a not-for-profit drug discovery and commercialization centre based at the Institute for Research in Immunology and Cancer (IRIC) of the Université de Montréal (UdeM). IRICoR's main objective is to rapidly translate highly innovative scientific projects from IRIC, UdeM and various centres into high value novel therapies in oncology, immunology and related indications through strong partnerships with the private sector — thereby efficiently bridging the innovation translation gap between early stage academic research and industry. For more information: www.iricor.ca

About the Centre for Commercialization of Regenerative Medicine (CCRM)

CCRM, a Canadian not-for-profit organization funded by the Government of Canada's Networks of Centres of Excellence program, the Province of Ontario, and leading academic and industry partners, supports the development of regenerative medicines and associated enabling technologies, with a specific focus on cell and gene therapy. A network of academic researchers, leading companies, strategic investors and entrepreneurs, CCRM aims to accelerate the translation of scientific discovery into marketable products for patients with specialized teams funding, and infrastructure. CCRM sources and evaluates intellectual property from around the globe, offers various consulting services, conducts development projects with partners, and establishes new companies built around strategic bundles of intellectual property. CCRM has a fully resourced, 6,000 square foot development facility used to both evaluate and advance technologies and 40,000 square feet in development for advanced cell manufacturing. CCRM is the commercialization partner of the Ontario Institute for Regenerative Medicine and the University of Toronto's Medicine by Design. CCRM is hosted by the University of Toronto and was launched in Toronto's Discovery District on June 14, 2011. Visit us at www.ccrm.ca

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