



I love stem cells

FROM MICROSCOPE TO STETHOSCOPE



CellCAN

Regenerative Medicine and
Cell Therapy Network

Have you ever been asked about stem cells or stem cell-based therapies in your practice?

Do you think stem cells and stem cell-based therapies are still only at a preclinical research stage?

Have you ever been confronted with a patient willing to pay thousands of dollars to get a treatment somewhere in the world?

Do you feel powerless with some of your patients living with chronic diseases?



WHILE THE MEDIA TALKS A GREAT DEAL ABOUT THE ENORMOUS POTENTIAL OF CELL THERAPY OR STEM CELL-BASED TREATMENTS, **THE REALITY IS EVEN MORE ENCOURAGING!**

Stem cells bring hope to several patients and physicians with the promise of offering new treatments for previously incurable diseases. Now with years of stem cell research, how far are we in realizing its full therapeutic potential?

DIFFERENT TYPES OF STEM CELLS AND THEIR PROPERTIES

EMBRYONIC STEM CELLS

They are pluripotent cells, which means that they can turn into any type of human cell. They are obtained from human embryos at a very early stage.

DID YOU KNOW...

A first bone marrow transplant (BMT) was performed more than 50 years ago, and it is estimated that over 50,000 bone marrow transplantations are done per year worldwide.

ADULT STEM CELLS

Adult stem cells are found in various tissues of the body. They can turn into a few different types of specialized cells. For example, hematopoietic stem cells, which are collected at the adult stage in the bone marrow, can give rise to all types of blood cells (white blood cells, red blood cells, and platelets).

INDUCED PLURIPOTENT STEM CELLS

Induced pluripotent stem cells are obtained by adding genes to adult stem cells (for example, the cells of the skin) to make them pluripotent, that is able to transform into any type of human cell. As they are created from the patient's own cells, they will not be rejected by the immune system after transplantation.

DID YOU KNOW...

Stem cells were discovered by Canadians James Edgar Till & Ernest McCulloch in 1963, at the University of Toronto!

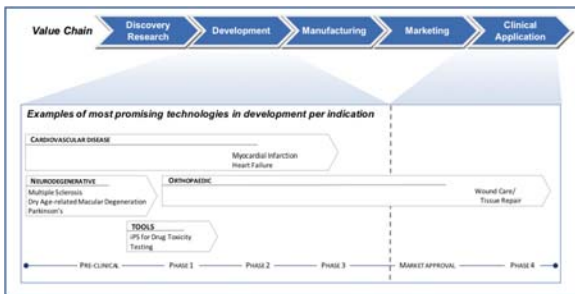
MESENCHYMAL STEM CELLS

Mesenchymal stem cells are found in bone marrow, grease, blood, placenta and umbilical cord blood. Like many other stem cells, they are able to migrate in the body to repair damaged tissue. However, contrary to other cells, they do not trigger immune reaction, i.e. they will not be rejected by the body after having been transplanted. In addition, they produce many molecules that help the surrounding cells to grow and regenerate. Further research needs to be done, but these unique properties could be very useful for the development of regenerative medicine.

ESTABLISHED THERAPIES

Hematopoietic stem cell transplantation (HSCT), known in the general public as bone marrow transplant (BMT), is a procedure consisting in transplanting hematopoietic stem cells derived from bone marrow, peripheral blood, or umbilical cord blood¹. HSCT is by far the stem cell therapy that is the most practiced. This procedure is most often performed on patients with leukaemia and lymphoma to reverse a side effect of conventional chemotherapy, which in addition to killing cancerous cells also destroys patients' stem cells within the bone marrow.

Other conditions treated with HSCT include sickle-cell disease, myelodysplastic syndrome, neuroblastoma, lymphoma, Ewing's sarcoma, desmoplastic small round cell tumor, chronic granulomatous disease, and Hodgkin's disease². Research on HSCT applications remains very active. As of February 2016, over 35% (1780/4941) of registered clinical trials on stem cell therapy were related to HSCT, targeting mainly leukemia and lymphoma patients³.



Source: Wang, T. (2013), Misuho Industry Focus: A Survey of Current Landscape in Regenerative Medicine. 141, 3-4

ONGOING CLINICAL TRIALS

Beyond established therapies, the number of clinical trials (CTs) has been on the rise since 2004 for an expanding range of conditions using new types of stem cells, including mesenchymal stem cells (MSCs), human embryonic stem cells (ESCs) or induced pluripotent stem cells (iPSCs).

The focus of novel stem cell CTs, with respect to disease indication, is cardiovascular disease. However, the immune system remains the most common target of stem cell therapies for various disease indications, including cancers, GvHD, and other non-malignant hematologic conditions, as well as autoimmune diseases such as multiple sclerosis, Crohn's disease and Type 1 diabetes.

Increasing numbers of CTs targeted neurological diseases, while CTs for liver disease, bone and cartilage conditions, diabetes, and eye diseases remained rare (<70 CTs globally).

Some types of study differ from the normal regulatory framework of clinical trials. These studies, called medical innovation, are exceptional and are performed by a doctor on a small number of very sick patients when he considers for scientific reasons that the results of preclinical research have demonstrated the treatment safety and efficacy and that it could be beneficial to patients.

It is important to note that medical innovation is not part of the regular framework of research development, but is a type of treatment available to doctors in special circumstances. As it is only available to a very small number of patients, clinical trials in due form need to be conducted by scientists or organizations before the treatment is approved and placed on the market.

UNPROVEN THERAPEUTIC CLAIMS

Many clinics worldwide offer stem cell treatments, but the efficacy and safety of these treatments have not always been scientifically proven or have not received regulatory or ethics approval. Some of these treatments can be harmful to patient health. Instead of scientific evidence, these clinics often use patient testimonials to demonstrate the effectiveness of their treatments. Indeed, sometimes patients want so badly for the treatment to be effective that they may feel it actually is. These effects, even if they are real, may not continue over time or may result from something other than the treatment. All of this is referred to as medical tourism.

DID YOU KNOW...

A promising technique, called “ex vivo expansion”, is the use of a molecule called UM171 that has the potential to multiply by 10 the number of cord blood units available for a transplant in humans; therefore addressing one of the major limitations of cord blood transplant to treat blood diseases.

Many patients who seek out cell therapy think they have nothing to lose, whether the therapy has been proven or not, but this isn't true. Therapies without any demonstrated efficacy or safety can still be dangerous, worsen the overall health of patients, and even put their lives in danger. Some patients who receive unproven treatments develop lesions, tremors, tumours or cancer, and these side effects can result in death. In addition, anesthesia and the transplant procedure itself also pose risks.

It is important to be aware of those practices because this could become a major hurdle for our health system in Canada. Also, patients should also know that receiving these treatments could prevent them from participating in legitimate clinical trials here in Canada, where eligibility criteria are very strict.

The time required to develop treatments can seem incredibly long to patients who have incurable or debilitating diseases. But scientists, doctors and regulators

are concerned about everyone's safety and well-being and must ensure that treatments will actually improve a condition and not cause additional problems (e.g., severe side effects). To expedite the availability of cell therapies, efforts are constantly made to improve and streamline the regulatory process.

CORD BLOOD AND STEM CELL BANKING

DID YOU KNOW...
Another reason why people undergo medical tourism treatments is for cosmetic reason (looking younger, baldness, etc.).

Considered medical waste up until 2004, cord blood is an incredible source of hematopoietic stem cells. Knowing that stem cells found in cord blood can potentially be used to treat more than 80 diseases and disorders – a number expected to increase up to 500 in 10 years from now – and how safe and simple the procedure of collecting cord blood is, one may consider to potentially saving a life, after giving birth, by donating umbilical cord blood.

In Canada on any given day, approximately 1,000 Canadians are in need for a stem cell donor. Among these, approximately 25% are able to find a match within their own family, which means 75% must receive stem cells from an unrelated donor, hence the relevance of cord blood banking.

One of the great advantages with cord blood is that complications like immune rejection and Graft versus Host Disease are less likely to occur with cord blood transplant. However, a limitation that is recognized with cord blood transplant is that more than one unit of cord blood is required to treat one adult (although one unit of cord blood may be sufficient to treat a child).

Public or Private Cord Blood Banking: What is the Difference?

Cord blood can be stored by public or private banks. A fundamental difference between the two storage options is that public cord blood banks store blood for the benefit of the general public, and private cord blood banks are usually for-profit organizations that store cord blood for the exclusive use of the donor or donor's relatives.

In Canada, the Canadian Blood Services (CBS) and Héma-Québec run public cord blood banks. In addition, Victoria Angel Registry of Hope, a charitable organization, picks up donor samples from about 35 hospitals throughout southern Ontario. Also, several research units accept cord blood that does not qualify for public cord blood banks. All public banks collect cord blood at no charge to the donor. Refer to CBS or Héma-Québec's websites for the list of partner hospitals.

¹ Karanes C, Nelson GO, Chitphakdithai P, Agura E, Ballen KK, Bolan CD, et al. (2008). Twenty years of unrelated donor hematopoietic cell transplantation for adult recipients facilitated by the National Marrow Donor Program. *Biology of Blood and Marrow Transplantation*. 14 (9 Suppl): 8–15.

² Worldwide Network for Blood & Marrow Transplantation (WBMT). Slides regarding 1 million transplants. <http://www.wbmt.org/en/general-information-by-laws-presentations/one-million-transplants/> Accessed February 22, 2016.

³ U.S. National Institutes of Health. Clinical trials [ClinicalTrials.gov](https://www.clinicaltrials.gov/ct2/home). <https://www.clinicaltrials.gov/ct2/home>. Accessed February 22, 2016.

ABOUT CELLCAN

CellCAN is a network that was created in April 2014 by Canadian researchers who work in regenerative medicine and cellular therapy.

Our mission: mobilize stakeholders and knowledge across Canada to significantly advance regenerative medicine and cell therapy research and clinical development.

CellCAN founding network affiliates include five Good Manufacturing Practice (GMP) compliant facilities which accommodate clinical grade production of human cell and tissue products. Together, CellCAN facilities equate to 30 Health Canada compliant clean rooms, able to do state of the art research and to treat patients across Canada. CellCAN's founding network affiliates include also two major research units in related areas or issues of ethics, law and regulation (ELR) and biotechnology.

CellCAN is also implementing platforms so that researchers, clinicians, granting bodies, industry stakeholders, charitable organizations, members of the government, patient representatives and the public can exchange knowledge, express their needs, and share their questions and even concerns.

ADDITIONAL RESOURCES

CellCAN's website: www.cellcan.com or www.ilovestemcells.ca

- Includes pamphlets for lay public on stem cells, medical tourism and the process of clinical trials.
- Includes a HelpDesk where you can ask specific questions and we'll put you in contact with the best person to answer (helpdesk@cellcan.com).

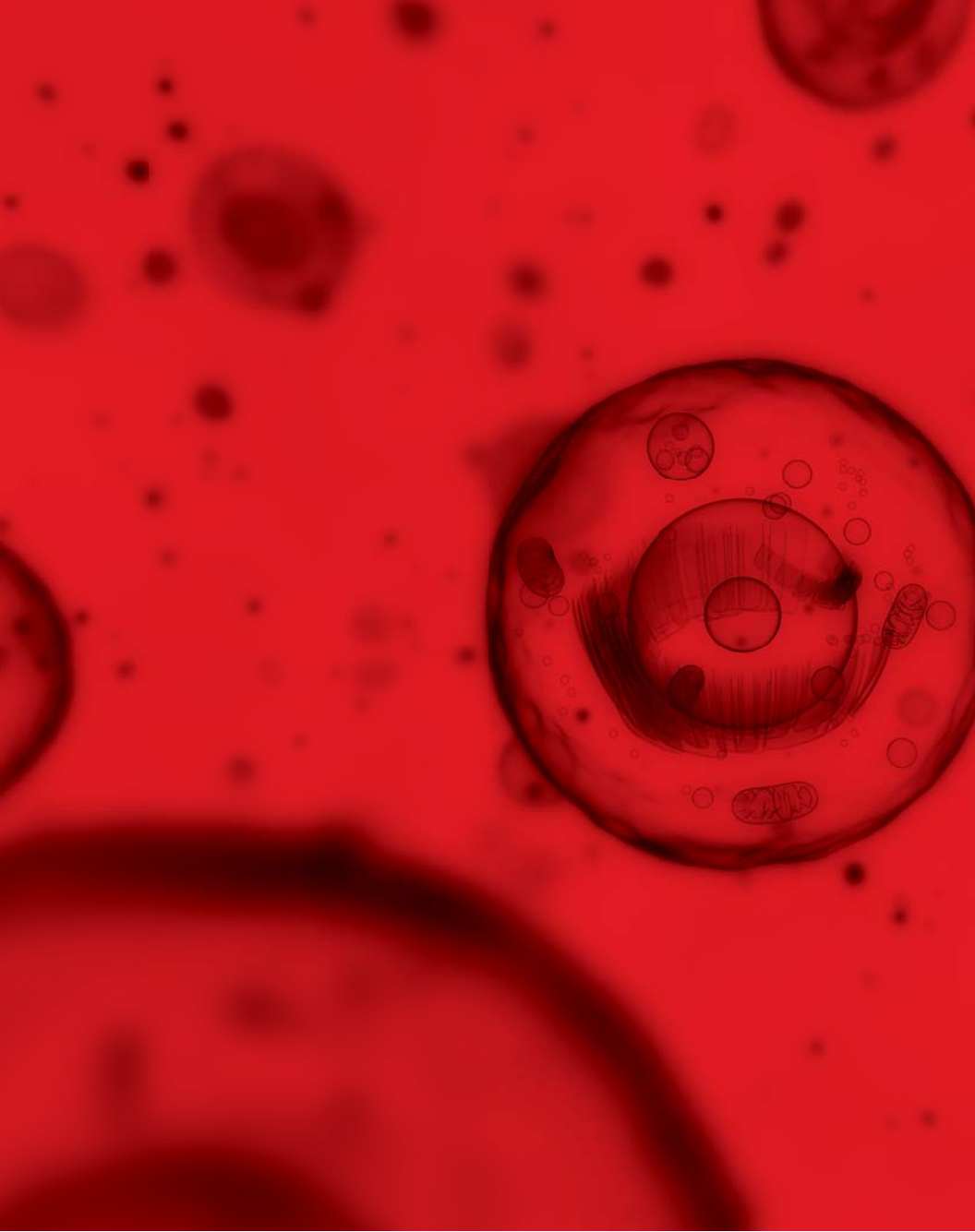
Reggie Mobile App – discover the potential and state of research for multiple diseases: www.cellcan.com/meet_reggie

Database on clinical trials: www.clinicaltrials.gov

CBS OneMatch and National Cord Blood Bank: www.blood.ca

Héma-Québec Stem Cell Registry and Cord Blood Bank:
www.hema-quebec.qc.ca

You can also refer to the Canadian Cancer Society (www.cancer.ca) or other charitable organizations on specific diseases for information on novel cell therapies offered.



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