Regenerative Medicine

What is Regenerative Medicine?

Regenerative medicine is a field of medicine that aims to treat human disease by replacing damaged tissue with cells or tissues created from stem cells or engineered biological materials. Currently, many chronic diseases can only be cured by organ transplantation. Since there is a shortage of donor organs, the medical field is in desperate need of a renewable source of cells and tissue for transplantation therapy. Regenerative medicine could provide an alternative to organ transplantation by using stem cells. Tissue resident stem cells such as hematopoietic stem cells of the bone marrow or specialized cells created from stem cells such as heart cells could be transplanted into patients to treat disease. Stem cells can also be used in the laboratory to generate tissue patches that can restore organ function when transplanted back into the body. Doctors and scientists hope that using regenerative medicine strategies will allow doctors to replace damaged tissue in more patients than the supply of donor organs currently allows.

How can stem cells be used to treat human disease?

Exogenous stem cell therapy: using stem cells from outside of the body to treat human disease

Cell replacement therapy

Bone marrow transplants (also known as blood or hematopoietic stem cell transplants) were the first stem cell therapy used to treat disease. Bone marrow transplants are used as part of the treatment for leukemia (a form of blood cancer) as well as other diseases that have damaged the function of the bone marrow, which is to generate new blood cells. Bone marrow contains undifferentiated blood stem cells. These stem cells have the ability to create all cell types of the blood, including red blood cells and platelet cells, but are not able to create other cell types. It is the presence of blood stem cells in the donor bone marrow that, when transplanted into the recipient, allow new specialized blood cells to be made for the entire lifetime of the recipient.

There are a number of sources of blood stem cells for transplantation. Blood stem cells can be used from a patient’s own tissue that has been stored in a freezer or blood stem cells from an immunogenetically matched donor (a donor that has the same tissue type as the patient). Markers known as human leukocyte antigens (HLAs) are found on most cells of the body which allow the body’s immune system to recognize the cells as its own. It is important to match patients and donors so that the patients’ body will recognize the transplanted cells as its own. Blood stem cells can also be found in umbilical cord blood. In recent years, new parents have been able to bank umbilical cord blood following the birth of their child. Umbilical cord blood has a higher concentration of blood stem cells than adult blood; however there is only enough blood in an umbilical cord to treat a small child.

Another cell replacement strategy is to introduce specialized cells created from stem cells into damaged or destroyed tissue. Many researchers around the world are testing methods of creating specialized cell types from pluripotent stem cells (See Stem Cell Differentiation). While the transplantation of undifferentiated hematopoietic stem cells through bone marrow transplants has been performed for many years, transplantation of specialized cells created from pluripotent stem cells is still in the research phase. The first FDA-approved human embryonic stem cell clinical trial began treating patients in late 2010. This research study aims to treat spinal cord injury by replacing lost myelin-producing cells with myelin-producing cells that have been generated from stem cells in the laboratory. Myelin is an
insulation layer around nerves that allows messages to be carried between the brain and other parts of the body. Scientists hope that transplanting new myelin-producing cells into a damaged spinal cord will restore the ability of the body to communicate with the brain.

What is tissue engineering?

Tissue replacement therapy
Regenerative medicine includes the field of tissue engineering. Tissue engineers aim to produce artificial tissue and organs from biological materials such as stem cells that can replace damaged tissue in the body. To create tissue patches in the laboratory, tissue engineers have incorporated cells into three-dimensional structures called scaffolds. Scaffolds are temporary support structures that recreate the natural shape and structure of tissues within the body. Scaffolds used for tissue engineering are made with biodegradable materials that will slowly degrade in the body after the patch has been transplanted. Scientists are currently testing the use of biodegradable scaffolds to generate a number of tissue types including bone, bladder muscle and heart muscle to treat disease. For example, heart cells that have been differentiated from stem cells can be used in the laboratory to make cardiac patches. Cardiac patches could be used to treat congestive heart failure. Congestive heart failure patients are not able to pump enough blood through the body because a portion of heart muscle has been damaged. Replacing damaged heart muscle with a tissue patch could restore function within the heart allowing it to deliver enough blood throughout the body.

What is personalized medicine and the role of iPSCs?

Induced pluripotent stem cells (iPSCs) and personalized medicine
iPSCs can be used in place of embryonic stem cells to obtain specialized cell types for therapies. iPSCs are pluripotent stem cells that have been generated from somatic cells such as a skin cell, and maintain the stem cell properties of embryonic stem cells (See Induced Pluripotent Stem Cells). The ability to create pluripotent stem cells from an individual’s own skin cells holds great promise for personalized medicine. Current transplantation therapies require immunogenetic matching between donor and recipient as well as the use of immunosuppressant drugs. Immunosuppressant drugs have a number of associated side effects, including the inability of the body to fight off infection due to the suppression of the body’s immune system. The first FDA-approved (USA Food and Drug Administration) human embryonic stem cell trial mentioned above; combines cell transplantation with immunosuppressant drugs. If a patient were to receive a transplant of specialized cells made from iPSCs from the patient’s own tissue (such as a skin sample), immunosuppressant drugs would not be required because the transplanted cells would already carry the mark of the patient’s tissue type. While iPSCs have the potential for generating customized cells and tissue for transplantation, research is still ongoing to determine their safety for use in treating human disease.

What are endogenous stem cells and can they be used to treat human disease?

Endogenous Stem Cell Therapy: Activating tissue resident stem cells to treat disease
Tissue resident stem cells are stem cells that are found deep inside tissues of the body. These stem cells are not usually dividing (also known as quiescent). Tissue resident stem cells have the ability to
generate all the cells of the tissue where they reside in the body. Endogenous stem cell treatments aim to activate tissue resident stem cells within a damaged tissue of the body to heal itself. Researchers have been studying the ability of drugs to instruct tissue resident stem cells to start dividing and differentiate into specialized cell types to replace damaged tissue. Human medical trials are currently underway to test the ability of tissue resident stem cells within the bone marrow to travel into the bloodstream to damaged parts of the brain following a stroke. These bone marrow stem cells then instruct tissue resident stem cells of the brain to divide and differentiate into new neurons. Stimulating endogenous stem cells already inside the body avoids a number of challenges with exogenous stem cell therapy including the possibility of immune rejection; surgical procedure risks and the potential for tumor formation (see Stem Cell Differentiation).