Stem Cell Therapy

Gene therapy (also called **human gene transfer**) is a medical field which focuses on the utilization of the therapeutic delivery of nucleic acid into a patient's cells as a drug to treat disease. The first attempt at modifying human DNA was performed in 1980 by Martin Cline, but the first successful nuclear gene transfer in humans, approved by the National Institutes of Health, was performed in May 1989. The first therapeutic use of gene transfer as well as the first direct insertion of human DNA into the nuclear genome was performed by French Anderson in a trial starting in September 1990. It is thought to be able to cure many genetic disorders or treat them over time.

In somatic cell gene therapy (SCGT), the therapeutic genes are transferred into any cell other than a gamete, germ cell, gametocyte, or undifferentiated stem cell. Any such modifications affect the individual patient only, and are not inherited by offspring. Somatic gene therapy represents mainstream basic and clinical research, in which therapeutic DNA (either integrated in the genome or as an external episome or plasmid) is used to treat disease.

As of 2016, the only established therapy using stem cells is hematopoietic stem cell transplantation (HSCT). This usually takes the form of a bone marrow transplantation, but the cells can also be derived from umbilical cord blood. Research is underway to develop various sources for stem cells as well as to apply stem-cell treatments for neurodegenerative diseases and conditions such as diabetes and heart disease.

About stem cells

Stem cells are undifferentiated, or "blank," cells. This means they're capable of developing into cells that serve numerous functions in different parts of the body. Most cells in the body are differentiated cells. These cells can only serve a specific purpose in a particular organ. For example, red blood cells are specifically designed to carry oxygen through the blood.

All humans start out as only one cell. This cell is called a zygote, or a fertilized egg. The zygote divides into two cells, then four cells, and so on. Eventually, the cells begin to differentiate, taking on a certain function in a part of the body. This process is called differentiation.

Stem cells are cells that haven't differentiated yet. They have the ability to divide and make an indefinite number of copies of themselves. Other cells in the body can only replicate a limited number of times before they begin to break down. When a stem cell divides, it can either remain a stem cell or turn into a differentiated cell, such as a muscle cell or a red blood cell.

Potential uses of stem cells

Since stem cells have the ability to turn into various other types of cells, scientists believe that they can be useful for treating and understanding diseases. Stem cells can be used to:

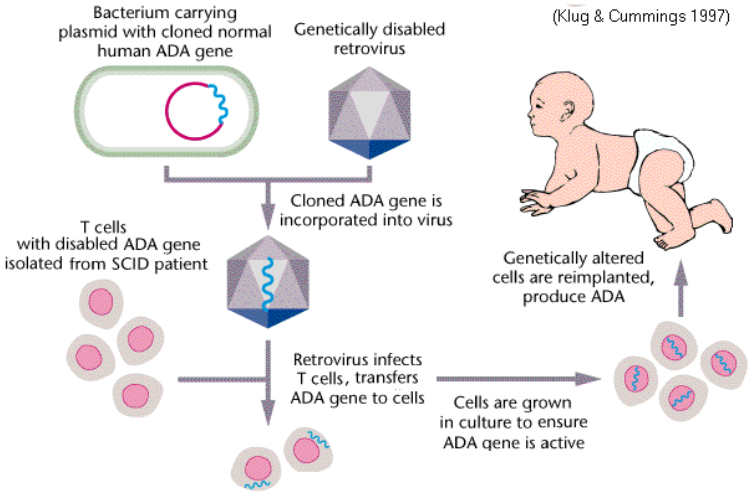
- grow new cells in a laboratory to replace damaged organs or tissues
- correct parts of organs that don't work properly
- research causes of genetic defects in cells
- research how diseases occur or why certain cells develop into cancer cells
- test new drugs for safety and effectiveness

Severe combined immunodeficiency (**SCID**) is a rare genetic disorder characterized by the disturbed development of functional T cells and B cells caused by numerous genetic mutations that result in differing clinical presentations. SCID involves defective antibody response due to either direct involvement with B lymphocytes or through improper B lymphocyte activation due to non-functional T-helper cells. Consequently, both "arms" (B cells and T cells) of the adaptive immune system are impaired due to a defect in one of several possible genes. SCID is the most severe form of primary immunodeficiencies.

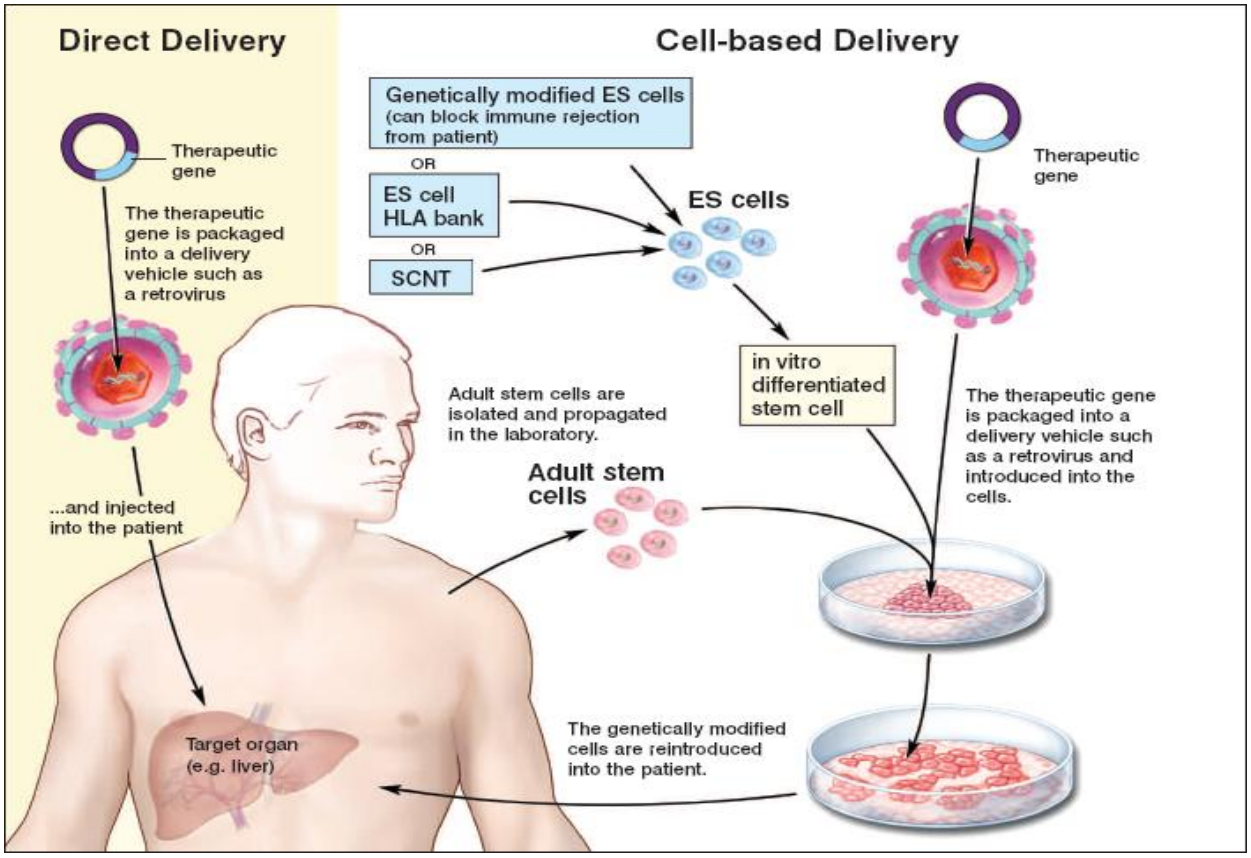
Allogeneic transplant is used for SCID. This type of transplant uses healthy, blood-forming cells (stem cells) donated by someone else to replace the unhealthy ones. These healthy cells can come from a family member, unrelated donor or umbilical cord blood. The cells create the immune system. First, the child gets chemotherapy

(chemo) to kill any unhealthy cells. Then, the healthy, donated cells are given to the child through an intravenous catheter. The new cells travel to the inside of the bones and begin to make healthy cells.

The entire transplant process, from the start of chemo until hospital discharge, can last weeks to months. This is followed by many months of recovery near the transplant center and at home. The transplant team will closely watch the child to prevent and treat complications.



Stem Cell Therapy to Cure SCID



Stem Cell Therapy For other diseases