



### Stem cells for curing human diseases

Stem cells are undifferentiated biological cells that can differentiate into specialized cells and can divide (through mitosis) to produce more stem cells. In mammals, there are two broad types of stem cells: embryonic stem cells, which are isolated from the inner cell mass of blastocysts, and adult stem cells, which are found in various tissues.<sup>[1,2]</sup> In adult organisms, stem cells and progenitor cells act as a repair system for the body, replenishing adult tissues.<sup>[1,2]</sup> In a developing embryo, stem cells can differentiate into all the specialized cells—ectoderm, endoderm and mesoderm but also maintain the normal turnover of regenerative organs, such as blood, skin, or intestinal tissues.<sup>[2]</sup>

There are three known accessible sources of adult stem cells in humans:

1. Bone marrow, which requires extraction by harvesting, that is, drilling into bone (typically the femur or iliac crest),
2. Adipose tissue (lipid cells), which requires extraction by liposuction, and
3. Blood, which requires extraction through apheresis, wherein blood is drawn from the donor (similar to a blood donation), and passed through a machine that extracts the stem cells and returns other portions of the blood to the donor.

Stem cells can also be taken from umbilical cord blood just after birth.<sup>[2]</sup>

Human stem cells are currently being used to test new drugs. New medications are tested for safety on differentiated cells generated from human pluripotent cell lines. Other kinds of cell lines have a long history of being used in this way. Cancer cell lines, for example, are used to screen potential anti-tumor drugs. The availability of pluripotent stem cells would allow drug testing in a wider range of cell types.<sup>[3]</sup>

Perhaps the most important potential application of human stem cells is the generation of cells and tissues that could be used for cell-based therapies. Today, donated organs and tissues are often used to replace ailing or destroyed tissue, but the need for transplantable tissues and organs far outweighs the available supply. Stem cells, directed to differentiate into specific cell types, offer the possibility of a renewable source of replacement cells and tissues to treat diseases including macular degeneration, spinal cord injury, stroke, burns, heart disease, diabetes, osteoarthritis, and rheumatoid arthritis.<sup>[3]</sup>

The use of embryonic and adult-derived stem cells for cardiac repair is an active area of research. A number of stem cell types, including embryonic stem (ES) cells, cardiac stem cells that naturally reside within the heart, myoblasts (muscle stem cells), adult bone marrow-derived cells including mesenchymal cells (bone marrow-derived cells that give rise to tissues such as muscle, bone, tendons, ligaments, and adipose tissue), endothelial progenitor cells (cells that give rise to the endothelium, the interior lining of blood

vessels), and umbilical cord blood cells, have been investigated as possible sources for regenerating damaged heart tissue.<sup>[3,4]</sup>

In people who suffer from type 1 diabetes, the cells of the pancreas that normally produce insulin are destroyed by the patient's own immune system. New studies indicate that it may be possible to direct the differentiation of human embryonic stem cells in cell culture to form insulin-producing cells that eventually could be used in transplantation therapy for persons with diabetes.<sup>[3,5,6]</sup>

Scientists have been able to do experiments with human embryonic stem cells (hESC) since 1998, when a group led by Dr. James Thomson at the University of Wisconsin developed a technique to isolate and grow the cells. Although hESCs are thought to offer potential cures and therapies for many devastating diseases, research using them is still in its basic stages.<sup>[7]</sup>

Late in 2007, scientists reported that they had been able to reprogram adult human skin cells to behave like hESCs. This type of stem cells is known as induced pluripotent stem cells, or iPSCs. Since these first reports, researchers have rapidly improved the techniques to generate iPSCs, creating a powerful new way to "de-differentiate" cells whose developmental fates were thought to be determined. And the Nobel Prize in Physiology or Medicine of 2012 was awarded jointly to Sir John B. Gurdon and Shinya Yamanaka "for the discovery that mature cells can be reprogrammed to become pluripotent".<sup>[7,8]</sup>

Scientists are working on the possible use of human spinal cord stem cells to treat Amyotrophic Lateral Sclerosis (ALS), also known as Lou Gehrig's Disease.<sup>[7,9]</sup> Along with this the work on use of adult mesenchymal cells (called Prochymal) for protecting pancreatic beta islet cells in adults and children with newly diagnosed type 1 diabetes, repair of heart tissue following a heart attack, and the repair of lung tissue in patients with chronic obstructive pulmonary disease (COPD) is also being done.<sup>[7]</sup>

Currently, scientists are testing whether limbal stem cells can help repair damage to the cornea and whether they can help replace cells that are lacking due to limbal stem cell deficiency.<sup>[7,10]</sup> The limbus is the marginal region of the cornea of the eye that contains stem cells. Stem cells from the limbus are called limbal stem cells, and they normally serve to replace cells to maintain the cornea. Limbal stem cells are being tested as possible treatments for human eye conditions.<sup>[7,10]</sup>

Scientists are testing the abilities of many different types of stem cells to treat certain diseases. Stem cells offer exciting promise for future therapies, and scientists are currently doing intensive research to overcome the hurdles that remain before stem cell therapy becomes a standard treatment.

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