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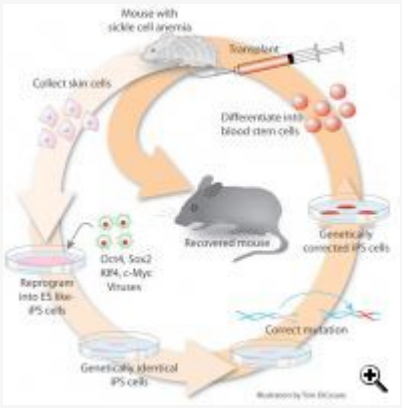
Induced Stem-Cells for Gene Therapy

Scientists from the Massachusetts Institute of Technology (MIT) and the University of Alabama at Birmingham succeeded in using induced stem-cells for gene therapy for the first time. The scientists used cells taken from the tip of a sickle-cell model mouse's tail to generate embryonic stem cells. The scientists genetically engineered the stem cells to cure the mouse's sickle-cell anemia. In the first stage, the cells were differentiated into hematopoietic cells (cells that generate red blood cells) and in the second stage they were transplanted back into the sick mouse. The procedure was successful and the mouse produced normal red blood cells. This experiment provided a first proof of concept for using induced stem cells as curing agents in gene therapy methods.

In several experiments conducted recently, skin cells were transformed into embryonic stem cell or into pluripotent cells, cells that can differentiate into any type of cell. Cells of this kind are called induced pluripotent stem (iPS) cells. The cells' transformation was achieved by changing the expression levels of four key regulators of their differentiating mechanisms. This overwhelming achievement opened up new therapeutic pathways for genetic diseases. The method of transforming the patient's own cells and transplanting them back into his or her body ensures that the body will not reject the implant and that the genetically engineered cells will be able to carry out their new function. Among others, the applications of this technology include introducing cells that can generate Insulin for diabetic people and regenerating brain cells in patients suffering from neuronal disorders like Alzheimer's disease or ALS.

The MIT and University of Alabama scientists transformed cells taken from the tip of a sickle-cell anemia model mouse's tail into hematopoietic cells capable of producing normal red blood cells. The first step in the process was transforming the skin cells into iPS cells. When the scientists managed to grow iPS cells, they used genetic engineering to fix the gene responsible for the sickle-cell anemia. In this way, they created healthy pluripotent cells, capable of differentiating into any desired cell type. After using a set of differentiating agents to turn the pluripotent cells into hematopoietic ones, the hematopoietic cells were implanted back into the mouse. Since the cells were originally taken from the same mouse, they weren't rejected by its body. Therefore, the cells were able to fulfill their goal and produce normal red blood cells.
The results of the experiment are promising. The introduced cells were detected in the mouse's blood stream even more than 12 weeks after the treatment. The engineered cells produced normal red blood cells, and about 70% of the red blood cells present in the mouse's blood stream were generated by them. The mouse showed normal behavior and did not suffer from any negative side effects. The scientists proved that iPS cells can be used as a therapeutic strategy in gene therapy treatment.

TFOT recently published some news articles regarding the successful use of stem cells in healing defected tissue. A breakthrough in the field was established when human stem cells were used to rebuild a rat's heart. Another breakthrough was when scientists successfully used stem cells to treat MS and ALS.



Mice received reprogrammed cells by tail injections
(Credit: MIT/Tom DiCesare)