Correcting sickle cell disease using stem cells

Science, Biology

Sickle cell anemia is a hereditary disease caused by the expression of hemoglobin S gene rather than that of hemoglobin A, producing crescent red blood cells (RBCs) instead of the normal concave RBCs. The abnormal shape prevents the proper transport of oxygen from the lungs to the other tissues of the body, and causes the RBCs to clump together, and clog the blood vessels. The disease manifests after 4 months of age, and its symptoms include painful episodes that last from hours to days, breathlessness, delayed growth, fatigue, fever, rapid heart rate, ulcers, jaundice, thirst, urination, priapism, and blindness (Zieve and Chen, 2011).

The pattern of inheritance of sickle cell anemia is autosomal recessive. That means the symptoms will only possibly manifest if both parents are carrier of hemoglobin A. However, at present, the only available long term treatment for the disease is bone marrow transplant, which is prone to rejection and subsequent immune system reaction. The goals of other treatments, blood transfusion and pain medicines, are only for short-term relief of symptoms (Zieve and Chen, 2011).

Recently, however, researchers from Johns Hopkins have found another plausible long term treatment for sickle cell anemia. By inserting the correct hemoglobin A gene to pluripotent bone marrow cells and removing the defective hemoglobin S, the patient can be able to produce RBCs that have the correct type of gene (Science Daily, 2011).

Despite this new breakthrough in treatment, this does not prevent inheritance of the disease, because the gene to be transferred to an offspring is contained in sex cells, and not the bone marrow cells that are to be fixed using the new technology recently developed.

References

Sicke cell anemia. Zieve, David and Chen Yi-Bin. Feb. 28, 2011. U. S. National Library of Medicine. October 27, 2011. Correcting Sickle Cell Disease with Stem Cells. Sep. 28, 2011. ScienceDaily. October 27, 2011. < http://www. sciencedaily. com/releases/2011/09/110928180416. htm>.