New Hope in Fight against Sickle Cell Anemia?

Sickle cell disease affects billions of people throughout the world and is particularly common among those whose descendants came from Sub-Saharan Africa; Spanish-speaking regions in the Western Hemisphere (South America, the Caribbean, and Central America); Saudi Arabia; India; and Mediterranean countries such as Turkey, Greece, and Italy. The natural course of the illness involves a complex cascade of events amalgamated with catastrophes often generated by infections.

Prophylactic antibiotics are a mainstay in an effort to stave off infection which can routinely catapult patients into a life-threatening crisis. The main oral manifestations of sickle cell anemia are mucosal pallor, yellow tissue coloration, radiographic abnormalities, delayed tooth eruption, disorders of enamel and dentine mineralization, changes to the superficial cells of the tongue, malocclusion, hypercementosis and a degree of periodontitis that is unusual in children. Mucosal pallor and yellowed discoloration of the gingiva result from the deposition of blood pigments secondary to hyperbilirubinemia caused by the large-scale destruction of erythrocytes. By early childhood, they develop a functional asplenia or ineffective spleen. Hence, they become especially prone to prodigious infection by encapsulated bacteria, and blood transfusions are frequent. Although the blood supply is well-tested for safety, recurrent transfusion can lead to issues like iron overload; this needs to be treated. The list goes on of the challenges, battles and treatment complexities these patients endure. Because fetal hemoglobin has a higher oxygen carrying capacity.

Sickle cell is a disease that is ripe for genetic advances for a few reasons. First, the gene that is affected is known and can be replaced by the healthy variant. Furthermore, the cells that are needed to be altered are easily accessible in the bone marrow. In many diseases, this is not the case. However, this one success story is incredibly encouraging for the sickle cell community and for moving the field of curing diseases using genetic editing forward.

The constant advancement of science, a cure may have been discovered for sickle cell disease. Doctors in Paris have monitored and confirmed the success of a DNA reversal treatment carried out on a teenager 15 months ago. Since the disease is such that the bone marrow produces deformed red blood cells, scientists altered the genetic directions in his bone marrow, so it produced healthy red blood cells. It is reported that the teenager had his bone marrow taken out, stem cells harvested and genetically

altered with a virus to infect it with correct instructions. Next, he underwent chemotherapy for 4 days to eliminate his diseased stem cells, before the corrected bone marrow was replanted. Since then, the teenager has received a clean bill of health. So far the patient has no sign of the disease, no pain, no hospitalization before the surgery, the condition of the teenager is said to have been critical. So much that he needed to have his spleen removed and his hips replaced. He also used to have a monthly blood transfusion to dilute his defective blood. But now, he no longer requires a transfusion.

Although Professor Leboulch is hesitant about asserting the treatment as a "cure" for sickle cell anemia, the success of the pilot case is a significant milestone in the treatment of the disease. According to the Professor, Leboulch there needs to be more performance of this therapy on several patients to create a certainty that it is "robust enough to propose it as a mainstream therapy."

However, just as the bone marrow transplant, the only known cure for sickle cell anemia is expensive, this pioneering treatment is quite expensive and can only be performed in highly advanced hospitals. This already poses a huge challenge to the Indian and African continent where the disease is predominant, as the cost implications are bound to widen between developed and developing countries to now if "cure" is in this gene therapy's future, much more data needs to be acquired, and study be implemented. Promising with cautious optimism might be the most apt description.

This is one of the classic genetic diseases everyone learns about in biology. As someone who works in the area of gene therapy every day, the realistic prospect of curing sickle cell disease with gene therapy is simply astonishing. The new treatment modality provides hope that the next generation of students will read about the devastation of this disease in history books only?

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Access this article online

Quick Response Code:

Website: www.contempclindent.org

DOI: 10.4103/ccd.ccd_275_17

How to cite this article: Damle SG. New hope in fight against sickle cell anemia?. Contemp Clin Dent 2017;8:1-2.