

Gene Therapy for Sickle Cell Disease

The Faulty Gene

Sickle cell disease occurs when a person inherits a faulty gene, which controls how red blood cells produce hemoglobin. Hemoglobin is the oxygen-carrying protein in red blood cells. Without enough healthy hemoglobin, red blood cells become stiff and sickled, which leads to inflammation and damage to blood vessels.



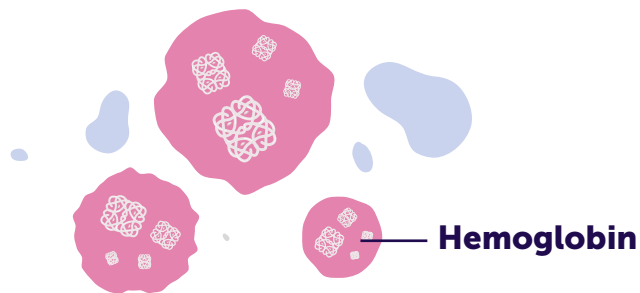
Healthy Red Blood Cells



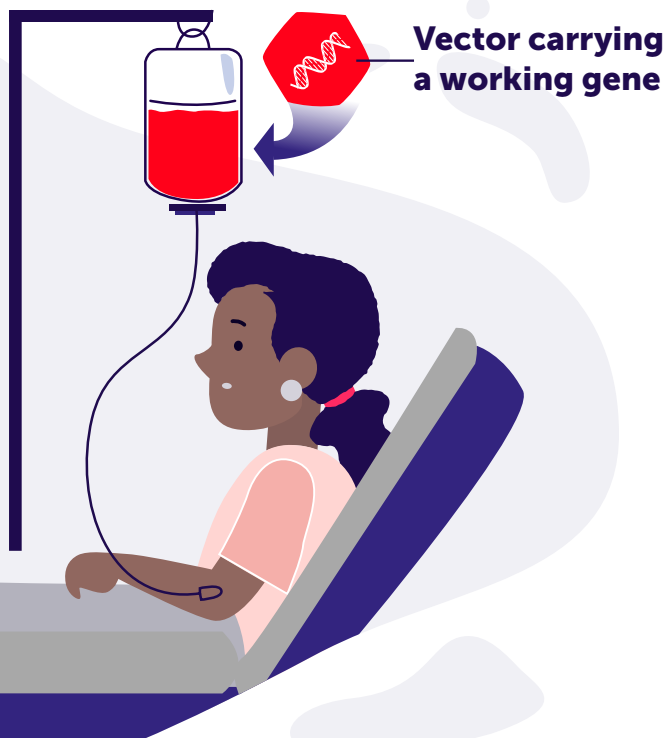
Sickled Red Blood Cells

Ex Vivo Gene Therapy

Gene therapy aims to slow or stop sickle cell disease by targeting the cause. This is done by introducing a working version of a gene that provides new instructions to the cells on how to make healthy red blood cells. The type of gene therapy used is called *ex vivo*, meaning that the person's own cells are removed from the body, modified with a new gene, and then returned.



Hemoglobin



Hemoglobin Production

There are two approaches used with gene therapy to target sickle cell disease. With the first approach, a new hemoglobin gene instructs blood-forming stem cells, called hematopoietic stem cells (HSCs), to produce healthy adult hemoglobin. In the other approach, the new hemoglobin gene can instruct the HSCs to silence, or switch off, a gene that blocks fetal hemoglobin. Switching off that gene causes an increase in fetal hemoglobin. Both of these approaches will make your body produce more healthy red blood cells and ultimately help you feel better.

The Role of Viral Vectors

The healthy hemoglobin genes are delivered to the cells using vectors, which are often derived from viruses. Viruses are well-known as a safe and effective way to get a new message inside cells. The harmful parts of the virus are removed, and the new message with the corrective treatment is delivered.

Any therapy options for sickle cell disease should be discussed with a health care provider.

For more information visit:

patienteducation.asgct.org

www.ashrc.org

www.hematology.org

Developed jointly with the NHLBI Cure Sickle Cell Initiative.