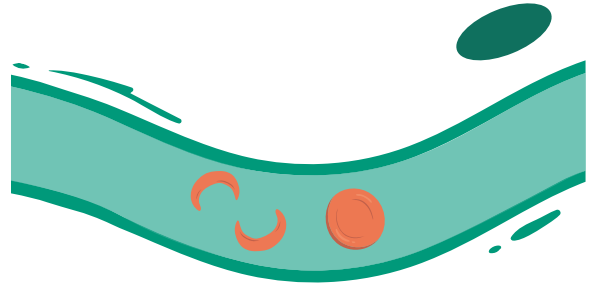
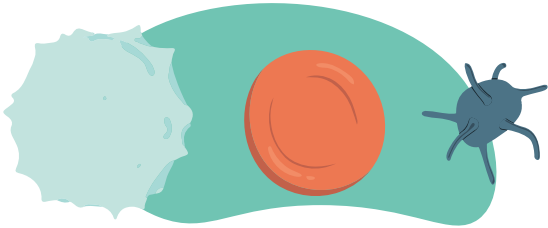


Gene Therapy for Blood Disorders

Gene therapy aims to target the cause of disease - a faulty gene - by providing working copies of the genes so blood cells function properly.

How Cells are Affected

A faulty gene can influence the way that blood cells are formed or how they function. For sickle cell disease the faulty HBB gene causes red blood cells to become rigid, resulting in slowed blood and reduced oxygen flow.



Using Stem Cells

Stem cells can form a variety of cells to serve different roles in our bodies. Hematopoietic stem cells, or HSCs, form our blood cells and can be used to treat blood disorders.

Remove Cells from the Body

HSCs are drawn from the patient's body and then modified. This process takes place outside the human body, which we refer to as *ex-vivo gene therapy*.



Vector Delivers New Gene

HSCs are modified using a vector - a virus without the disease causing parts - to deliver a working gene to do the job that the faulty genes cannot.

Return to the Body

Once the modified cells are returned to the patient, they go back to work making blood cells that supply oxygen, fight infection, and control blood clotting.

