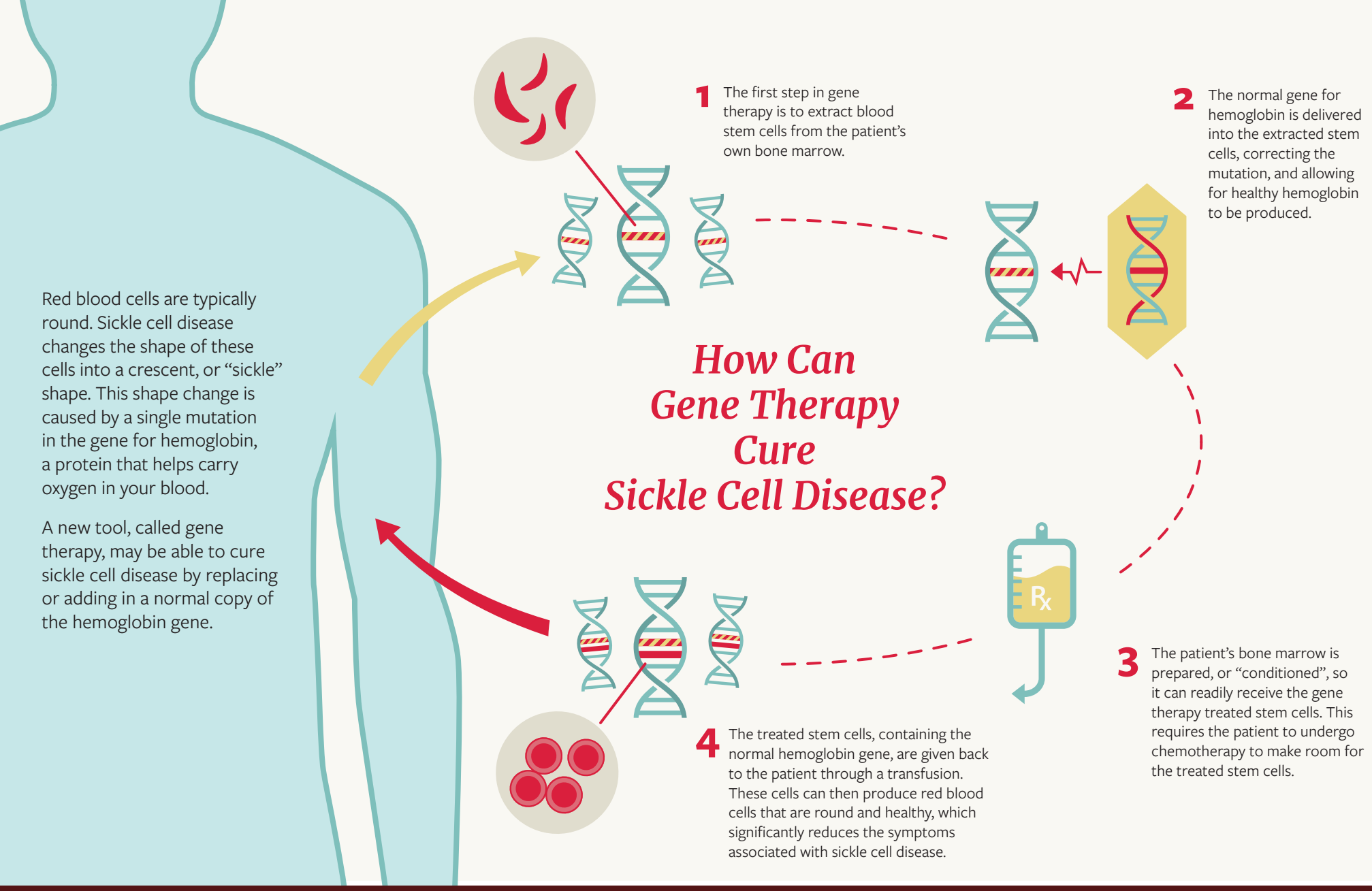
How Can Gene Therapy Cure Sickle Cell Disease?

Red blood cells are typically round. Sickle cell disease changes the shape of these cells into a crescent, or "sickle" shape. This shape change is caused by a single mutation in the gene for hemoglobin, a protein that helps carry oxygen in your blood.

A new tool, called gene therapy, may be able to cure sickle cell disease by replacing or adding in a normal copy of the hemoglobin gene.



1 The first step in gene therapy is to extract blood stem cells from the patient's own bone marrow.

2 The normal gene for hemoglobin is delivered into the extracted stem cells, correcting the mutation, and allowing for healthy hemoglobin to be produced.

3 The patient's bone marrow is prepared, or "conditioned", so it can readily receive the gene therapy treated stem cells. This requires the patient to undergo chemotherapy to make room for the treated stem cells.

4 The treated stem cells, containing the normal hemoglobin gene, are given back to the patient through a transfusion. These cells can then produce red blood cells that are round and healthy, which significantly reduces the symptoms associated with sickle cell disease.

CURE SICKLE CELL.
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The advantages to a gene therapy approach include decreased chance for rejection, decreased need for blood transfusions and pain medications, and the possibility of a permanent cure. If you would like to know more about participating in a clinical trial, please speak with your physician, call the NHLBI Center for Health Information at 1-877-NHLBI4U (1-877-645-2448), or visit ClinicalTrials.gov.

National Heart, Lung, and Blood Institute