Genetic Therapies

Genetic therapies are the newest therapies for Sickle Cell Disease:

Exagamglogene autotemcel (Casgevy) is a gene therapy that uses CRISPR gene editing to silence a gene in red blood cells. This enables the body to produce a fetal form of hemoglobin, which does not cause cells to sickle.

Lovotibeglogene autotemcel (Lyfgenia) is a one-time gene therapy to treat sickle cell disease in patients 12 years of age or older and a history of vaso-occlusive events. LYFGENIA is made specifically for each patient, using the patient's own blood stem cells (from which red blood cells are produced). It adds functional copies of the beta-globin gene to your cells leading to production of anti-sickling hemoglobin that may decrease or stop vaso-occlusive events.

While there is no universal cure for sickle cell disease (yet), there are several therapeutic approaches to relieve symptoms, reduce complications, and extend life. Early treatment (by eight weeks) by a hematologist is critical for newborns. To Learn more about these treatments, you should follow up with your sickle cell provider.

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SICKLE CELL DISEASE TREATMENTS

Sickle cell disease is an inherited blood disorder. Normal red blood cells are smooth and flexible discs, like the letter O, enabling them to move easily through blood vessels. In people with sickle cell disease, sickle cells are stiff, sticky, and often shaped like the letter C. Sickle cells tend to cluster together and to the lining of blood vessels, making it difficult for them to move through small blood vessels. These clusters can create blockages in small blood vessels, stopping the movement of healthy, oxygen-carrying blood. This blockage decreases oxygen delivery to the tissues, which can cause pain and organ dysfunction and is what causes the majority of the complications of the disease.

What are the treatment options for sickle cell disease in children and teens?

Penicillin

In children who have sickle cell disease, taking penicillin two times a day has been shown to reduce the chance of having a severe infection caused by the pneumococcus bacteria. Newborns need to take liquid penicillin. Older children can take tablets. Typically, children with sickle cell disease take penicillin until the age of 5 years in order to prevent pneumococcal infections.

Recommended Immunizations

Immunizing children with sickle cell disease requires special considerations due to their increased susceptibility to infections and the unique challenges presented by this condition. Children with sickle cell disease should follow a special vaccination schedule.

Children with sickle cell disease should receive a variety of vaccines, including pneumococcal .

- Pneumococcal: Protects against Streptococcus pneumoniae, which can cause pneumonia and blood infections .
- Flu: Protects against severe flu complications
- Meningococcal: Protects against Neisseria meningitidis.
- Hepatitis B: Protects against hepatitis B.

Preventing Dehydration, drink plenty of fluids, particularly during hot weather . Dehydration increases the risk of a sickle cell crisis. Drinking plenty of water daily (8 to 10 glasses) and avoiding dehydration.

PAIN

Hydroxyurea Therapy

Hydroxyurea is an oral medication that causes the body to produce fetal hemoglobin (HbF), a type of hemoglobin normally found only in fetuses and very young children. Increasing the healthy fetal hemoglobin can significantly reduce the side effects and complications of sickle cell disease.

Transfusion Therapy

Blood transfusions may be given acutely in order to treat severe anemia, acute chest syndrome, and other life-threatening complications of sickle cell disease. Children with sickle cell disease may receive chronic blood transfusions to prevent complications such as stroke.

L-glutamine: An oral powder that can help reduce the frequency of pain crises. L-glutamine is an FDA-approved oral treatment for sickle cell disease (SCD) in patients 5 years and older, shown in a Phase 3 trial to reduce vaso-occlusive crises (VOCs), hospitalizations, and acute chest syndrome. It functions by increasing NADH, which helps sickle red blood cells remain flexible and reduces their adhesion to blood vessel walls.

Crizanlizumab (Adakveo): An injectable medication that can help reduce the frequency of pain crises in adults and children older than 16. Side effects include nausea, joint pain, back pain, and fever. (Adakveo remains fully approved in the U.S. for treating SCD patients ages 16 and older)

Deferiprone (Ferriprox): A medication that binds to iron and removes it from the bloodstream. It is used to treat iron overload caused by blood transfusions.

Bone Marrow Transplant

Bone marrow transplant (BMT) can cure sickle cell disease. Most BMTs done for sickle cell disease are currently performed in children who have had complications such as strokes, acute chest crises, and recurring pain crises.

These transplants usually use a matched donor. Blood and bone marrow transplants are riskier in adults.

BMT is not a surgery. BMT involves replacing stem cells in the bone marrow with new stem cells that will make healthy red blood cells. The following are types of BMT for sickle cell disease, matched sibling donor transplant, and alternative donor including haplo-identical (parent) transplant.