BMT CTN
Blood & Marrow Transplant Clinical Trials Network

RESEARCH NEWS

Transplant helps teens, young adults with sickle cell disease

Longer, larger studies are needed to show best treatment

A small study showed blood or marrow transplant (BMT) is one good option for treating sickle cell disease.

Sickle cell disease is an inherited disease or passed down from parents to children. Normal red blood cells are round and flexible. But in sickle cell disease, the cells are stiff and can block the flow of blood. These stiff sickle cells cause episodes of severe pain and can harm the brain, causing strokes.

BMT can cure sickle cell disease but can have serious effects.

The standard care for sickle cell disease includes medicines and blood transfusions. These help with short-term relief. People get the standard care more often than BMT. Gene therapy is another option now approved for sickle cell disease. But, very few persons have received gene therapy.

Researchers wanted to compare standard care versus BMT. About 110 patients with sickle cell disease joined a clinical trial, starting in 2016. Everyone was aged 15-40. Patients who had a matched BMT donor got BMT; patients with no donor got standard care.

The trial ended early because not enough patients joined, and also the COVID-19 pandemic prevented patients from coming for treatment.

About 2 years after treatments started, about 9 out of 10 patients were alive, whether they got standard care or matched BMT. However, the trial was not able to determine which treatment helped people live longer.

Compared to patients who got standard care, the patients who got BMT:

- Had fewer episodes of severe pain
- Felt less tired
- Were more able to be social with friends and family

Keep in mind

BMT sometimes has serious effects, such as graft-versus-host disease (GVHD) and organ damage.

This study was smaller and shorter than planned. Larger, longer studies are needed to show which treatment is best.

Learn more about

- Sickle cell disease at NMDP.org
- Clinical trials for sickle cell disease at CTsearchsupport.org
- More study summaries at CIBMTR.org

This plain-language summary (PLS) was written by Jennifer Motl at Medical College of Wisconsin and reviewed by an author of the full article.

Source

Walters MC, Eapen M, Liu Y, et al. Hematopoietic Cell Transplant compared with Standard Care in Adolescents and Young Adults with Sickle Cell Disease. Blood Advances. 2024. Epub 2024 Oct 29. doi: 10.1182/bloodadvances.2024013926.

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Clinical Trial IDs

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