

Half-matched transplant helps adults with sickle cell disease

Certain medicines make the transplant safer

A clinical trial has shown that a blood or marrow transplant (BMT) can cure sickle cell disease in adults. BMT for sickle cell disease has been studied more in children and with fully matched transplants. This trial, using newer medicines, showed that BMT also helps adults.

Severe sickle cell disease causes crippling pain, strokes, and organ damage.

BMT can cure sickle cell disease, but it sometimes causes infections, graft-versus-host disease (GVHD), and other serious effects. In the past, people could only get BMT if they had a fully matched donor. But with newer medicines, people can get BMT from a half-matched donor, like a parent or sibling.

The trial included 42 teens and young adults who had severe sickle cell disease. Everyone got BMT during 2017 to 2021, plus:

- Low-dose total body irradiation
- 4 medicines to prepare for BMT: anti-thymocyte globulin, cyclophosphamide, fludarabine, and thiotepea
- 3 medicines to prevent GVHD: cyclophosphamide, mycophenolate mofetil, and sirolimus.

About 2 years after BMT:

- 90% of patients were alive with no sickle cell disease or effects
- 10% of patients were very sick or died

During the study, 2 patients died:

- 1 patient died of sickle cell disease before BMT
- 1 patient died of COVID-19 about 2 years after BMT

Keep in mind

The results of this trial are similar to an earlier trial. Both trials showed that BMT with a half-matched donor plus newer medicines for GVHD can cure sickle cell disease in adults. Ask your doctor about treatments.

Source


Kassim et al. [Haploidentical Bone Marrow Transplantation for Sickle Cell Disease](#). New England Journal of Medicine Evidence. 2025;4(3): EVIDoA2400192. Epub 2025 Feb 25. PMC11932095.


Learn more about

- [Sickle cell disease](#) at NMDP.org
- [Clinical trials of 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. © 2025 by CIBMTR, license CC BY-SA 4.0.



 **90% of patients were alive with no sickle cell disease or effects**

 **10% of patients were very sick or died**

Sponsor

Blood and Marrow Transplant Clinical Trials Network® (BMT CTN®) BMT CTN thanks study participants.

BMT CTN, funded by the National Heart, Lung, and Blood Institute and by the National Cancer Institute, is a collaborative effort of 19 core transplant centers/Consortia, the Center for International Blood and Marrow Transplant Research (CIBMTR), NMDP, and The Emmes Company. CIBMTR is a research collaboration of the Medical College of Wisconsin (MCW) and NMDP.

Clinical Trial IDs

BMT CTN 1507; ClinicalTrials.gov NCT03263559



BMT CTN Data Coordinating Center

Center for International Blood and Marrow Transplant Research® • Medical College of Wisconsin
Clinical Cancer Center, 9200 W. Wisconsin Avenue, Suite C5500, Milwaukee, WI 53226 • 414-805-0700

Learn more at [BMTCTN.net](#).