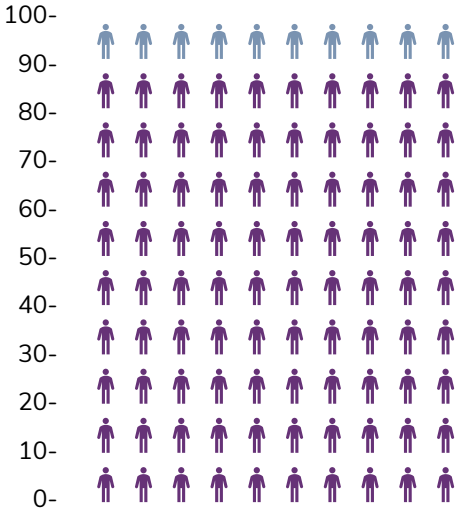
Half-matched transplant helps adults with sickle cell disease

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 thiotepa
- 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.

Learn more about

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

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.

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Learn more at BMTCTN.net

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