

## New risk score predicts person's chance that transplant will work to treat sickle cell disease

### Children who have matched sibling donors are helped most

Blood or marrow transplant (BMT) can cure sickle cell disease for some people. Now, doctors can predict which people will benefit most from BMT.

### What is sickle cell disease?

People with sickle cell disease have a gene that makes C-shaped red blood cells, instead of round cells. The C-shaped cells get stuck in small blood vessels and cause pain, strokes and other problems.

### BMT sometimes cures sickle cell disease

Before BMT, a person's own blood and marrow cells are wiped out by medicines. BMT replaces these cells with healthy cells donated by another person. When BMT works, the healthy cells stay and the person is cured of sickle cell disease.

However, sometimes the healthy cells don't grow like they should in the bone marrow, and the person isn't cured.

### Who should get a transplant for sickle cell disease?

Researchers studied the medical records of about 1,400 people who got BMT for sickle cell disease. Any of these 3 things raised the likelihood that BMT would work to cure the sickle cell disease:

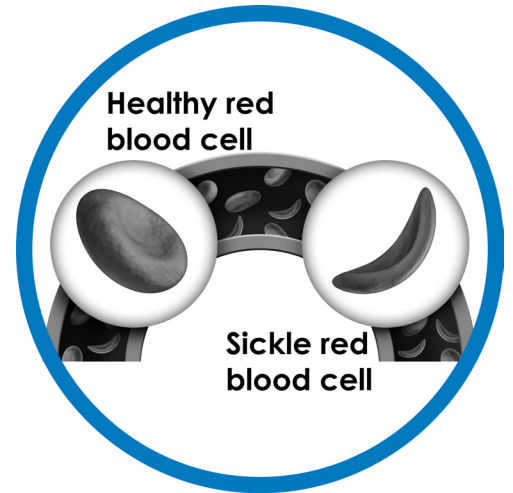
- Patient was aged 12 or younger during transplant
- Donor was the patient's brother or sister (people with sickle cell trait can still donate for someone with sickle cell disease)
- Donor was well matched (had similar genes) to the patient

Using complex math, doctors developed a risk score to predict who would be healthiest after BMT. The risk score can help people with sickle cell disease and their families make treatment decisions.

For example, researchers found BMT was most helpful for children who got BMT when they were aged 12 or younger if their disease was severe enough to need a BMT and whose donor was a matched brother or sister. Three years after BMT, 92% (9 out of 10) of these children were alive and had healthy cells.

### Ask your doctor

- What is my risk score?
- What are the potential benefits and harms of BMT and other treatments?



### Learn more about

- [Sickle cell disease at BeTheMatch.org](http://BeTheMatch.org)
- [Clinical trials at CTSearchSupport.org](http://CTSearchSupport.org)
- [More study summaries at CIBMTR.org](http://CIBMTR.org)

### About this research summary

This information is provided on behalf of the Consumer Advocacy Committee of the CIBMTR<sup>®</sup> (Center for International Blood and Marrow Transplant Research<sup>®</sup>).

### Source

Brazauskas R, Scigliuolo GM, Wang HL, et al. [Risk score to predict event-free survival after hematopoietic cell transplant for sickle cell disease.](#) Blood. 2020 Jul 30;136(5):623-626. doi: 10.1182/blood.2020005687. PMC7393258.

