BMT CTN
Blood & Marrow Transplant Clinical Trials Network®

RESEARCH NEWS

Most children are alive and well after unrelated donor transplant for sickle cell disease

Blood or marrow transplant from unrelated donors is an option

Blood or marrow transplant (BMT) can cure sickle cell disease. However, less than 1 in 5 children have a tissue-type matched, sibling donor who doesn't have sickle cell disease.

Researchers wanted to know if matched, unrelated donors could also be helpful. A small clinical trial during 2008-2014 included 21 children with sickle cell disease who received BMT from matched unrelated donors. About 8 years after BMT:

- 68% (2 out of every 3 children) were alive
- 57% (6 out of every 10 children) were healthy

However, some children had very serious problems related to BMT, including graft-versus-host disease (GVHD), where the transplanted cells attack the child's organs. These complications were more common in children older than 13 years at the time of transplant. In one child, the transplanted cells did not grow.

A few children and teens had organ problems that could have been caused either by sickle cell disease or BMT or both. The organs affected were the adrenal glands, sex organs and pancreas. Some patients had problems with depression and anxiety.

Keep in mind

A matched sibling donor, when available, is still the best donor, with excellent outcomes. However, if needed, an unrelated, matched donor can be an option.

What's next

More research is needed and is currently underway to prevent GVHD after transplant. Ask your doctor about preventing GVHD.

Learn more about

- Sickle cell disease at NMDP.org
- Clinical trials for SCD 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

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Clinical Trial IDs

MT CTN 0601; ClinicalTrials.gov NCT00745420

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Learn more at BMTCTN.net.