Half-matched transplant helps people with aplastic anemia

New treatment combination helps children, adults of all races and ethnicities

People of all races and ethnicities can get successful transplants for severe aplastic anemia (SAA), according to a new study.

SAA is a rare but serious blood disorder. Children and adults with SAA get very sick with low blood counts, infections or bleeding.

If SAA does not respond to the first choice of therapy or comes back (relapses) after a period of health, then we call this relapsed and refractory SAA. Blood or marrow transplant (BMT) is the only cure for relapsed and refractory SAA.

Family members or unrelated volunteers can donate cells if they match with the patient. Many people do not have a fully matched donor, though, to have a BMT. A new study shows that a haploidentical, or half-matched, BMT from a family member can help people live longer.

This especially helps people who are American Indian or Alaska native, Asian, Black or African American, Native Hawaiian, other Pacific Islander, more than one race, or Hispanic. It's easier for people in these communities to find a related half-matched than a fully matched unrelated BMT donor.

For this study, 31 people with relapsed and refractory SAA got half-matched transplants. People had a variety of racial and ethnic backgrounds: Less than half of the people in this trial were white.

A year after BMT, more than 80% of people were alive. Few people had chronic graft-versus-host disease (GVHD), a possible side effect of BMT. People's health-related quality of life was normal.

Researchers said 3 things helped control SAA. First, use enough bone marrow cells from the donor for the transplant itself. Second, reduced-intensity preparations are enough chemo prior to the BMT. Third, cyclophosphamide reduces the risk of GVHD enough to use a half-matched donor.

Keep in mind

Ask your doctor about the short- and long-term effects of BMT on your body.

Learn more about

- Severe aplastic anemia at BeTheMatch.org
- Clinical trials for SAA at CTsearchsupport.org
- More study summaries at CIBMTR.org

Original research article

DeZern AE, Eapen M, Wu J, et al. Haploidentical bone marrow transplantation in patients with relapsed or refractory severe aplastic anaemia in the USA (BMT CTN 1502): a multicentre, single-arm, phase 2 trial. Lancet Haematology. 2022;9(9):e660-e669. Epub 2022 July 31. PMC9444987. doi: 10.1016/s2352-3026(22)00206-x.

Clinical Trial IDs: BMT CTN 1502; ClinicalTrials.gov NCT02918292

Sponsor: Blood and Marrow Transplant Clinical Trials Network (BMT CTN)

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About this research summary

The BMT CTN is a collaboration of The Center for International Blood and Marrow Transplant Research (CIBMTR); The Medical College of Wisconsin; The National Marrow Donor Program /Be The Match; and The Emmes Company.

Learn more at BMTCTN.net