

Experimental medicine treats children with leukemia and MDS

Treosulfan may be safer than a similar medicine before transplant

Treosulfan may be safer than a similar medicine used to prepare children for blood or marrow transplant (BMT), according to new research.

Scientists studied 40 children, aged 1 to 19, who had blood cancers. The children had either acute myeloid leukemia (AML) or myelodysplastic syndrome (MDS). BMT sometimes cures these cancers.

To prepare for transplant, children got radiation and chemotherapy (chemo) medicine to kill the cancer cells.

Some common types of chemo used for transplant, such as a drug called busulfan, can make people very sick. A new chemo, treosulfan, does not seem to have as many side effects as busulfan. But, treosulfan is not yet approved in the U.S. It is used in Europe to treat ovarian cancer and it is being studied in Europe for bone marrow transplant as well.

In this study, instead of busulfan, doctors gave the children treosulfan. The children in this study had fewer side effects than would have been expected with busulfan, scientists said. For example, with treosulfan, no children had severe liver or bladder problems.

And treosulfan worked as well as busulfan to control cancer. It also prevented graft-versus-host disease (GVHD) equally well.

This study was a joint effort of the Pediatric Blood and Marrow Transplant Consortium and the Center for Blood and Marrow Transplant Research (CIBMTR), and took place at several hospitals across the U.S.

Keep in mind

Right now, in the U.S., treosulfan is offered only in clinical trials. More research is needed.

Ask your doctor

What is the best way for me, or my child, to prepare for BMT? What medicines will I, or my child, get in the days before transplant?

Learn more about

- [This research, from its publication](#)
- [Transplant for children and teens, from Be The Match](#)
- [This trial, NCT01772953, on ClinicalTrials.gov](#)

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Source

Nemecek ER, Hilger RA, Adams A, et al. Treosulfan, fludarabine and low-dose total body irradiation for children and young adults with acute myeloid leukemia or myelodysplastic syndrome undergoing allogeneic hematopoietic cell transplantation: A prospective phase II trial of the Pediatric Blood and Marrow Transplant Consortium. *Biology of Blood and Marrow Transplantation*. 2018 Aug; 24(8): 1651–1656. doi:10.1016/j.bbmt.2018.04.025. Epub 2018 May 9. PMC6108922.

About this research summary

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