

Stem cell transplantation for children with rare form of leukemia improves outcomes

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Researchers in the Division of Hematology, Oncology and Blood & Marrow Transplantation at Children's Hospital Los Angeles have shown greatly improved outcomes in using stem cell transplantation to treat patients with a serious but very rare form of chronic blood cancer called juvenile myelomonocytic leukemia (JMML).

Allogeneic hematopoietic [stem cell transplantation](#) (HSCT) involves the transplantation of stem cells from a donor, which may be derived from bone marrow, peripheral [blood](#) or umbilical cord blood. The recipient's immune system is usually destroyed with radiation or chemotherapy before the transplantation. It is the only reported cure for JMML; however best outcomes of the therapy have shown only that half of patients can be cured from their disease. According to the researchers, there is currently no standard conditioning regimen for children with JMML undergoing HSCT.

The CHLA study, led by Hisham Abdel-Azim, MD, looked at children with JMML who underwent HSCT at Children's Hospital Los Angeles. All of the patients were alive and in clinical remission.

"The lack of transplant-related mortality in the group of children we studied at the Children's Center for Cancer and Blood Diseases at CHLA suggests that BUMEL (Intravenous Busulfan and Melphalan) may represent a successful HSCT high-dose chemotherapy regimen," said Abdel-Azim. "It is also possible that administering conventional dose chemotherapy, before HSCT, to patients with more progressive disease

may have contributed to the improved outcomes."

He added that a follow up clinical trial is warranted to confirm these promising findings, which are published in the July 23, 2015, issue of the journal *Blood*.

Provided by Children's Hospital Los Angeles

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