

Patients with treatment-resistant CLL respond positively to stem cell transplants

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Allogeneic (donor-derived) stem cell transplant (alloSCT) may be a promising option for patients with treatment-resistant chronic lymphocytic leukemia (CLL), regardless of the patient's underlying genetic abnormalities, according to the results of a study published online today in *Blood*, the journal of the American Society of Hematology. About 15,000 new CLL cases were diagnosed in the United States in 2009 and about 4,000 deaths were documented (according to the American Cancer Society). While survival rates for leukemia have generally improved in the last decade, patients with rare, more aggressive forms of CLL do not respond well to standard chemotherapy-based and targeted treatments and often die within a few years of diagnosis.

Patients with CLL who are treatment-resistant (do not respond to chemotherapy and targeted antibody combination regimens) have been shown to have genetic abnormalities that predict their lack of response. In this study, researchers investigated whether alloSCT could be an effective treatment for this patient population, independent of underlying genetic abnormalities.

"This study, which is one of the largest of its kind, confirms that allogeneic stem cell transplants are a promising therapeutic option for treatment-resistant CLL patients fighting particularly aggressive disease, regardless of their genetic risk profile," said Peter Dreger, MD, of the Department of Medicine, University of Heidelberg, Germany, and lead author of the study. "However, because stem cell transplants come with



serious risks, they should be reserved for only this group of patients until further studies can be done."

In alloSCT, blood stem cells are collected from a donor and then infused into the patient where they travel to the bone marrow and begin to produce new blood cells, replacing those that have been affected as a result of the disease. This type of treatment can pose serious complications, some of which are potentially fatal. In this prospective phase II study, a total of 90 patients with treatment-resistant CLL received alloSCT, and stem cell donors were either healthy siblings or unrelated, but matched, volunteers.

Prior to the transplant, patients in this study received conditioning, a standard therapy administered immediately before a <u>stem cell transplant</u> to help prepare the body to receive and accept the transplanted cells. The research team used a reduced-intensity conditioning approach with two common chemotherapies (fludarabine and cyclophosphamide) to reduce complications and allow the donor stem cells to fight the disease themselves.

After treatment with alloSCT, more than 40 percent of participants with this otherwise fatal disease enjoyed long-term freedom from relapse. These findings suggest that alloSCT is a feasible and potentially curative treatment for patients with high-risk CLL and should be considered for this patient population.

Provided by American Society of Hematology

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