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Stem Cell Transplantation in Thalassemia

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ABSTRACT The beneficial results of stem cell transplantation from HLA identical family members for patients with severe thalassemia are clear. Class I patients have a very high probability of cure with a very low early and late morbidity and mortality. Delay of transplantation until the patient is in a risk category beyond class I substantially reduces the probability of transplant success and jeopardizes the reversibility of liver and cardiac damage. It is reasonable to suggest that patients with b-thalassemia who have HLA-identical donors should be transplanted as soon as possible. Umbilical cord blood (UCB) has been shown to be capable of reconstituting the bone marrow of the patient with thalassemia after myeloablated pre-conditioning treatment. The major advantage of UCB over other sources of stem cells is the ability to cross HLA barriers, and there is evidence of less GVHD. The use of related –donor UCB stem cells with HLA mismatches at one to three antigens needs to be considered. It would be worth while to do a prospective study to evaluate the role of UCB stem cell transplantation in the treatment of the thalassemias and hemoglobinopathies.

Home Back