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Allogeneic Bone Marrow Transplantation for P-Thalassemia Major

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Abstract

Patients suffering from B-thalassemia major, a relatively common hemoglobinopathy found in children of southern Chinese origin, require frequent red blood cell transfusions to survive. This causes progressive hemosiderosis and is inevitably fatal. Although intravenous chelation therapy with desferrioxamine has enabled these patients to survive into adulthood, it also carries a number of significant adverse effects such as epiphyseal dysplasia, auditory, visual and neurotoxicity. Non-compliance is common in the adolescent years. Bone marrow transplantation (BMT) is the only known cure for the disease. From 1988 to 1994, our center has performed ten allogenic BMT on patients suffering from B-thalassemia major. The patients ages between 0.8 to 13.4 years (mean=7.15 years). Five out of ten (50%) patients are ethnically Chinese. Patients with high serum ferritin (>2000ug/1) (n=2) were given high dose desferrioxamine infusion to lower their ferritin levels. Preparative regimen consisted of Cyclophosphamide 200mg/kg IV and Busulphan 14-16mg/kg PO. All patients were given Cyclosporine A and IV Methotrexate for graft versus host disease (GVHD) prophylaxis.

Results: All the patients survived bone marrow transplantation. With a follow up period of 1.3 to 7.3 years (mean=4.3 years), eight out of ten patients (80%) had sustained engraftment with permanent hematological correction. Four out of ten patients (40%) had mild to moderate acute GVHD and two out of ten patients (20%) showed evidence of mild chronic GVHD.

Nine out of ten (90%) patients have good functional status with Lansky scores of 100%. All the patients with successful engraftment and their parents expressed the opinion that BMT is the treatment of choice for the disease. Our result compares favorably with other BMT series on B-thalassemia major and confirms its role for the treatment of such patients with an HLA-identical sibling. This series constitutes one of the largest series on Chinese patients.