



Allogeneic hematopoietic stem cell transplantation in children with aplastic anemia

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ABSTRACT. The aim of this study was to prospectively investigate the efficacy and safety of fully matched allogeneic hematopoietic stem cell transplants in children with severe aplastic anemia in China. A total of twenty patients with severe aplastic anemia were enrolled in our study. Thirteen cases underwent transplantation with fully human leukocyte antigen (HLA)-matched, granulocyte-colony stimulating factor (G-CSF)-primed bone marrow and peripheral blood stem cells (PBSCs) from matching sibling donors. One patient received fully HLA-matched bone marrow from an unrelated donor. Six patients received fully HLA-matched G-CSF-primed PBSCs from unrelated donors. The conditioning regimen included fludarabine, cyclophosphamide, and rabbit anti-thymocyte globulin. Graft-versus-host disease prophylaxis was conducted with cyclosporin A and short-course methotrexate. The median follow-up duration was 3.08 years (range, 0.83-8.41years). The median time of neutrophil recovery ($>0.5 \times 10^9/L$) was 14 days (range, 10-20 days), and the median time of platelet recovery ($>20 \times$

$10^9/L$) was 19 days (range, 14-31 days). The survival rate at the cutoff point of follow-up was 95.0% (19/20). Initial engraftment rate was 95% (19/20). Late graft failure (graft failures occurring 1 year or longer after transplantation) was observed in one patient. Only one patient developed Grade I acute graft-versus-host disease. Two cases suffered from Epstein-Barr virus (EBV)-associated post-transplant lymphoproliferative disorder and remitted after treatment with rituximab. One patient was diagnosed with hyperthyroidism 2.5 years after transplantation. Our study indicated that allogeneic hematopoietic stem cell transplantation is an effective and safe treatment for children with severe aplastic anemia in China.

Key words: Allogeneic hematopoietic stem cell transplantation; Pediatric; Acquired aplastic anemia