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Allogeneic Hematopoietic Stem Cell Transplantation for Children with Severe Aplastic Anemia

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Abstract

Objective: Allogeneic hematopoietic stem cell transplantation (allo-SCT) is a potentially curative treatment for severe aplastic anemia (SAA). This is a single institutional review to study the feasibility of using allo-SCT for Thai children with SAA.
Material and Method: Nine children with SAA (7 matched-sibling donor-SCT, 1 matched-unrelated donor-SCT and 1 haploidentical-SCT) underwent allo-SCT between October 2002 and September 2010. Cyclophosphamide and anti-thymocyte globulin (CY/ATG) were used as conditioning regimen for 4 patients with matched-sibling donor-SCT. CY/ATG and fludarabine were used for 3 patients with matched-sibling donor-SCT and one patient with haplo-identical SCT. One matched-unrelated donor-SCT received CY/ATG and total body irradiation.
Results: Eight of 9 patients (89%) achieved neutrophil engraftment within 13.5 days (range 6.0-22.0). One matched-sibling donor-SCT recipient who failed to achieve engraftment died from acute renal failure and gram-negative sepsis on day 21 post allo-SCT. One matched-sibling donor-SCT case developed late graft failure on day 72 and died from invasive fungal infection. For graft versus host disease (GVHD), a haplo-identical-SCT patient died from steroid refractory grade IV acute GVHD. At last follow-up, six patients (67%) alive at a median follow-up time of 76.4 months (range 2.3-88.8). Overall survival (OS) and event-free survival (EFS) at 5 year was 63% and 65%, respectively.
Conclusion: Allo-SCT is a feasible curative treatment for children with SAA in Thailand. Graft failure and severe GVHD in alternative donors SCT are responsible for major causes of death. OS and EFS probabilities are stable after the first year post transplant.

Keywords: Allogeneic stem cell, Stem cell transplantation, Aplastic anemia, Children

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