Stem Cell Transplantation for Severe Aplastic Anaemia in Children: A Single Institute Experience

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Abstract

Fifteen children diagnosed of severe aplastic anaemia were treated with allogeneic haematopoietic stem cell transplantation (HSCT). The patients presented at the median age of 8.5 years, and 7 of 15 patients received HSCT within 2 months after diagnosis. The other patients had received immunosuppressive treatment but failed to response, they were then subsequently transplanted at 8-208 months after diagnosis. Ten patients had Human Leukocyte Antigen (HLA) identical sibling HSCT, whereas the other 5 patients received 1-3 antigen mismatched family donors (n=4) or unrelated donor transplant (n=1). Except two patients, all the other patients had successful engraftment of donor cells at median of 19 days. Those with successful engraftment did not have significant complications, and 5/13 patients developed graft versus host disease which responded to immunosuppressive treatment. Two patients without engraftment subsequently died of infection. The overall survival for the whole group was 86%, and it was 100% for HLA identical or mismatched sibling transplant. At a median follow up of 7 years, the survivors were all in good health without late morbidity. In conclusion, HLA identical sibling HSCT achieved a high chance of cure and is the treatment of choice for children with severe aplastic anaemia.

Key words

Aplastic anaemia; Immunosuppressive therapy; Stem cell transplantation

Introduction

Severe aplastic anaemia (SAA) is a rare disease in children with an incidence of 2-3 per million paediatric population. Most of the cases are idiopathic without underlying causes, but the pathogenesis is thought to be immune mediated. Before the era of potent immunosuppressive treatment, SAA carried a high morbidity and mortality rate. With the introduction of anti-lymphocyte globulin or anti-thymocyte globulin and cyclosporine, the survival is now improved to 70% at 5 years after diagnosis. However some patients only have partial response or relapse after initial response. Some patients are dependent on long term immunosuppressive treatment. The long term disease free survival after immunosuppressive treatment is about 50-60%. Allogeneic bone marrow transplantation from a Human Leukocyte Antigen (HLA) compatible sibling is the preferred treatment option because it is associated with a high cure rate. We report the result of allogeneic haematopoietic stem cell transplantation (HSCT) for SAA in a single institute over 15 years.

Patients and Method

Patients were diagnosed acquired SAA if they fulfilled the following criteria: (1) severe anaemia that was transfusion dependent, (2) absolute neutrophil counts <0.5 x 10^9/L, (3) platelet count <20 x 10^9/L, (4) a hypoplastic bone marrow on biopsy with cellularity <5%. There should