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Preliminary Program

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Hematopoietic stem cell transplantation from alternative donors for patients with Fanconi anemia and Diamond-Blackfan anemia: a single center experience

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Abstract

Aim: To analyze the outcomes of allogeneic hematopoietic stem cell transplantation (allo-HSCT) from alternative donors on Fanconi anemia (FA) and Diamond-Blackfan anemia (DBA).

Methods: Between February 2007 and June 2011 six patients with FA and three patients with DBA underwent alternative donor allo-HSCT. The median patient age was 9.5 years (range 3.7–18.5). Eight patients received allo-HSCT from matched unrelated donors. Haploidentical donor allo-HSCT was performed in one patient with FA-associated acute myeloid leukemia. A non-myeloablative fludarabine-containing conditioning regimen was used for all patients. All patients were transfusion-dependent at the time of HSCT.

Results: Complete donor engraftment was achieved in 6 patients. One patient with DBA had initial autologous hematopoietic recovery, which was followed by a second transplant that resulted in 50% donor chimerism. Acute and chronic GVHD occurred in 5 and 2 patients respectively. Six patients are alive and well with a median follow-up of 11 (range 1–29) months after transplant. The causes of death of 3 patients were hemorrhage, acute GVHD grade IV (gut, liver), and transplant-related toxicity.

Conclusions: Our small study confirms encouraging results of alternative donor allo-HSCT for patients with FA and steroid-resistant transfusion-dependent DBA.

Keywords: Fanconi anemia, Diamond-Blackfan anemia, allogeneic hematopoietic stem cell transplantation, alternative donor

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