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Non-T-cell depleted haploidentical HSCT after RIC in pediatric malignancies

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Abstract

The main purpose of this study was to evaluate the potential outcomes of haploidentical hematopoietic stem cell transplantation (HSCT) with reduced intensity conditioning regimen (RIC) in the management of pediatric solid and hematological malignancies.

Our protocol, based (mostly) on fludarabine, ATG, and busulfan, was used in 40 pediatric patients with refractory hematological (n=28) or solid (n=12) malignancies. Diagnoses were: AML: 10, ALL: 4, CML: 4, JMML: 5, MDS: 1, NHL: 4, NB: 7, Ewing's S: 4, and melanoma: 1. The median age of patients (pts) was 8.5 yrs (1–18). HLA compatibility was: 3/6: 62.5%, 4/6: 27.5%, and 5/6: 10%. In vitro graft T-depletion with vincristine and methylprednisolone was the only procedure performed.

Three pts with leukemia progression at the time of transplantation didn't recover and died. Four pts with JMML/MDS rejected and relapsed less than 2 months after transplantation. Thirty-three pts (82.5%) recovered, and achieved full donor chimeras after transplantation. Toxicity was mild in all but one case. Incidence of acute GVHD II–IV in first 100 days was 53% (gr IV=0). Incidence of chronic GVHD was 52%. Relapse rate 1 yr after transplantation was 75% and 33% for solid tumors and hemoblastoses respectively. In two pts with hemoblastoses the second transplantation was successful. In the entire group the results were as follows: 9 pts (22.5%) now alive, with median follow-up of 41 months (8.1–88.5); 20 (50%) pts died due to relapse; and 11 (27.5%) died of other causes: 2 pts (5%) of acute GVHD, 7pts (17.5%) of chronic GVHD, and 2 pts (5%) of infection.

Haploidentical HSCT after RIC in children can provide long-term anti-leukemia/lymphoma effect without significant complications. Patients with JMML/MDS and solid tumors need additional therapeutic modalities.

Keywords: anti-tumor effect, reduced intensity, haploidentical hematopoietic stem cell transplantation, children