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POSTER ABSTRACTS

203.LYMPHOCYTES AND ACQUIRED OR CONGENITAL IMMUNODEFICIENCY DISORDERS

Successful Treatment Outcomes of Hematopoietic Stem Cell Transplantation with Reduced-Toxicity Conditioning Regimen Incorporating Treosulfan in Pediatric Patients with XIAP Deficiency

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Background
X-linked inhibitor of apoptosis (XIAP) defciency is an inherited primary immunodefciency characterized by chronic infammasome overactivity and associated with hemophagocytic lymphohistiocytosis (HLH) and infammatory bowel disease (IBD). Although hematopoietic stem celltransplantation (HSCT) is the only curative therapy, the outcomes of HSCT for XIAP deficiency remain unsatisfactory.
Methods
We have performed HSCT with treosulfan based RTC regimen for pediatric patients with non-malignant disorders since January 2016, and reviewed the medical records of patients with XIAP deficiency who underwent treosulfan based HSCT to investigate outcomes of HSCT for patients with XIAP deficiency.
Results
Since January 2016, 4 patients with XIAP deficiency had undergone HSCT using RTC regimen consisted of fludarabine (150-180 mg/m ^{2}), treosulfan (42 g/m ^{2}), rabbit ATG (5-7.5 mg/kg), and thiotepa (10 mg/kg). All patients were male and received HSCT (2 from URD and 2 from HFD) at median 13.7 years old (range, 12.8-15.3), median 2 years (range, 0.5-3.9) after diagnosis of XIAP. They all achieved engraftment and complete donor chimerism at the time of last evaluation. One patient who received HFD HSCT developed acute GI GVHD grade 3 and VOD within 1 month from HSCT, which was recovered after treatment with systemic steroid and defibrotide. Except the patient, there was no fatal acute regimen related toxicity $\geq$ CTCAE grade 3. Two patients developed chronic GVHD: one had limited disease which was resolved after systemic steroid therapy and the other had extensive disease. During the median 43 months (range, 33.1-63.0) follow-up, all survived without disease recurrence.
Conclusions
HSCT with treosulfan based RTC regimen is promising treatment option for pediatric patients with XIAP deficiency, with successful engraftment and low treatment-related toxicity. However, further studies including a larger multicenter trial and measures to refine the regimen are mandate to verify efficacy and safety of this regimen.

Disclosures No relevant conflicts of interest to declare.

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