Improved outcome of treatment-resistant high-risk Langerhans cell histiocytosis after allogeneic stem cell transplantation with reduced-intensity conditioning.

Children with multisystem Langerhans cell histiocytosis (LCH) and risk organ involvement who fail to respond to conventional chemotherapy have an extremely poor prognosis. Myeloablative stem cell transplantation (SCT) as a possible salvage approach for these patients has been associated with a high risk of transplant-related mortality. Therefore, allogeneic stem cell transplantation following a reduced-intensity conditioning regimen (RIC-SCT) has recently been performed as an alternative salvage approach. We report on the experience with allogeneic RIC-SCT in nine pediatric high-risk LCH patients. Conditioning regimen included fludarabine in all patients, melphalan in eight patients, total lymphoid irradiation in six patients, total body irradiation in two, antithymocyte globulin in five, and Campath in four patients. RIC-SCT was well tolerated with regard to common procedure-related complications. Two patients died 50 and 69 days after RIC-SCT, respectively. Seven out of the nine patients survived and showed no signs of disease activity (including one with nonengraftment and full autologous hematopoietic recovery) after median follow-up of 390 days post-SCT. Based on this observation, we conclude that RIC-SCT is a
feasible procedure with low transplant-related morbidity and mortality and a promising new salvage approach for high-risk LCH patients with resistant risk organ involvement.

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