Preemptive immunotherapy in childhood acute myeloid leukemia for patients showing evidence of mixed chimerism after allogeneic stem cell transplantation.

Previous studies have shown that children with acute myeloid leukemia (AML) who developed mixed chimerism (MC) were at high risk for relapse after allogeneic stem-cell transplantation (allo-SCT). We investigated the feasibility of intensified preemptive immunotherapy in children receiving allo-SCT for AML. Eighty-four children were registered in our trial from May 2005 to April 2009; of these, 71 fulfilled the inclusion criteria and were treated according to the study protocol. Serial and semiquantitative analyses of posttransplantation chimerism were performed. Defined immunotherapy approaches were considered in MC patients. Continuous complete chimerism (CC) was observed in 51 of 71 patients. MC was detected in 20 patients and was followed by immunotherapy in 13. Six of 13 MC patients returned to CC without toxicity and remained in long-term remission. Overall, the probability of event-free survival (pEFS) was 66% (95% confidence interval [95% CI] = 53%-76%) for all patients and 46% (95% CI = 19%-70%) in MC patients with intervention; however, this number increased to 71% (95% CI = 26%-92%) in 7 of 13 MC patients on immunotherapy who were in remission at the time of transplantation. All MC
patients without intervention relapsed. These results suggest that MC is a prognostic factor for impending relapse in childhood AML, and that preemptive immunotherapy may improve the outcome in defined high-risk patients after transplantation.