

# Measurable residual disease-based strategy and reduced-intensity hematopoietic stem cell transplantation for acute myelogenous leukemia in children

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## Abstract

**Background.** Measurable residual disease (MRD) is a powerful prognostic factor in patients with acute myelogenous leukemia (AML). We previously conducted a prospective study on an MRD-based strategy for the treatment of children with AML, and its significance has been reported. The present retrospective study is a pooled analysis of 34 patients who were registered after the completion of the previous study. **Procedure.** Reduced-intensity conditioning (RIC) followed by allogeneic hematopoietic stem cell transplantation (RIC-HSCT) was adopted to increase survival rates and minimize toxicities. The strategy was also refined with a minor modification, with WT1 expression levels in peripheral blood, as an MRD marker after a 2nd course of chemotherapy (consolidation course 1), being measured in addition to WT1, chimeric mRNA, and aberrant surface markers in bone marrow. **Results.** Five-year relapse-free and overall survival rates were 76.5 and 85.2%, respectively. RIC-HSCT was safely and effectively performed on MRD-positive patients. Among patients who underwent RIC-HSCT, re-emerging/rising MRD and high levels of MRD before HSCT were risk factors for disease relapse early after HSCT. **Conclusions.** The MRD-based strategy and RIC-HSCT worked well for children with AML. HLA-haploidentical peripheral blood transplantation following RIC may be a promising candidate for further study on patients at very high risk of relapse.

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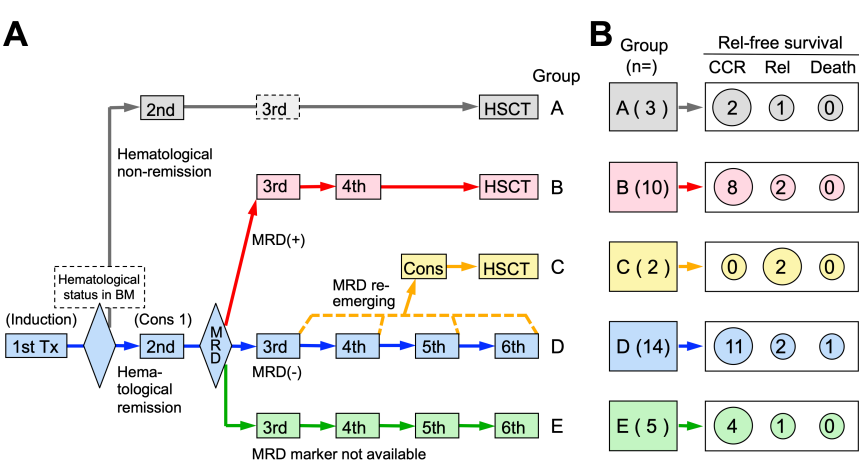


Figure 1

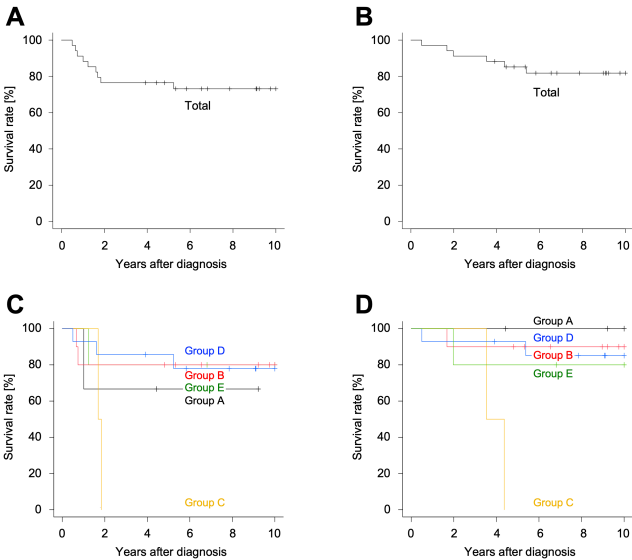


Figure 2