Encouraging outcome of children with beta-thalassemia major who underwent fresh cord blood transplantation from an HLA-matched sibling donor

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Abstract

Background: Allogeneic hematopoietic stem cell transplantation(allo-HSCT) is currently the only curative treatment for thalassemia major (TM). Cord blood (CB) from a sibling has different characteristics from marrow and has potential advantages and disadvantages as a stem cell source. Methods: We retrospectively analysed 68 children with β-TM who underwent fresh cord blood transplantation (F-CBT) from an human leukocyte antigen (HLA)-matched sibling donor (MSD) between June 2010 and July 2018 in the Department of Pediatrics, Nanfang Hospital and Haematology-Oncology, Shenzhen Children's Hospital. Results: The median infused doses of total nucleated cells (TNCs) and CD34+ cells were 8.51×107/kg and 3.16×105/kg,respectively. The median time to neutrophil and platelet engraftment were respectively 27 days and 31 days. The cumulative probability of acute and chronic graft- versus-host disease (GVHD) were very low after F-CBT (7.8% and 0.0%, respectively). Of the 68 paediatric patients, 67 patients survived during a median follow-up period of 61 months.The estimated 5-year probability of overall survival (OS) and disease-free survival (DFS) were 98.5% and 92.4%, respectively. Three patients experienced graft rejection (GR) (4.5%) , and this study found that GR was higher in the thiotepa (TT)-free regimen group than that in the TT-based regimen group (0% vs.10.7%,P=0.038). Multivariable prognostic analysis, a conditioning regimen including TT, improved the DFS of patients with β-TM receiving F-CBT (P=0.032). Conclusions: The above results indicate that patients with β-TM have excellent outcomes after F-CBT from an HLA-MSD.

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