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Title: HLA-Haploidentical Stem Cell Transplant with Pre-Transplant Immunosuppression for Patients with Sickle Cell Disease

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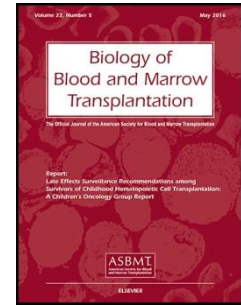
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HLA-Haploidentical Stem Cell Transplant with Pre-transplant Immunosuppression for Patients with Sickle Cell Disease

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Highlights

- Stem cell transplant can offer a cure for patients with sickle cell disease (SCD)
- SCD patients often lack suitable stem cell donors and have high rate of graft failure
- Successful haploidentical transplant significantly expands the donor pool for SCD
- PTIS with a reduced toxicity regimen provides prompt engraftment and low toxicity

Abstract

Allogeneic stem cell transplantation (HCT) is curative in patients with severe sickle cell disease (SCD) but a significant number of patients lack a HLA-identical sibling or matched unrelated donor. Mismatched related (haploidentical) HCT with post-transplant cyclophosphamide (PTCY) allows expansion of the donor pool but is complicated by high rates of graft failure. In this report, we describe a favorable haploidentical HCT approach in a limited cohort of sickle cell patients with significant co-morbidities. To reduce the risk of graft failure we administered the conditioning regimen of rabbit anti-thymocyte globulin (ATG), busulfan (BU) and fludarabine (FLU) preceded with two courses of pre-transplant immunosuppressive therapy (PTIS) with FLU and dexamethasone (DEX). Graft-versus-host disease (GVHD) prophylaxis consisted of PTCY on

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