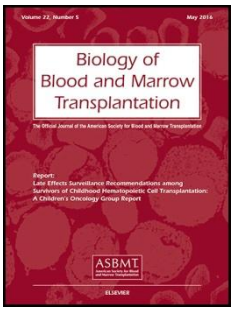
Accepted Manuscript



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

Author: Anna B. Pawlowska, Jerry C. Cheng, Nicole A. Karras, Weili Sun, Leo D. Wang, Alison D. Bell, Lisa Gutierrez, Joseph Rosenthal

PII: S1083-8791(17)30694-8
DOI: http://dx.doi.org/doi: 10.1016/j.bbmt.2017.08.039
Reference: YBBMT 54791

To appear in: *Biology of Blood and Marrow Transplantation*

Received date: 2-6-2017
Accepted date: 30-8-2017

Please cite this article as: Anna B. Pawlowska, Jerry C. Cheng, Nicole A. Karras, Weili Sun, Leo D. Wang, Alison D. Bell, Lisa Gutierrez, Joseph Rosenthal, HLA-Haploidentical Stem Cell Transplant with Pre-Transplant Immunosuppression for Patients with Sickle Cell Disease, *Biology of Blood and Marrow Transplantation* (2017), http://dx.doi.org/doi: 10.1016/j.bbmt.2017.08.039.

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

Anna B Pawlowska[1], Jerry C Cheng[2], Nicole A Karras[1], Weili Sun[1], Leo D Wang[1], Alison D Bell[1], Lisa Gutierrez[1], Joseph Rosenthal[1].

[1] Department of Pediatrics, City of Hope, Duarte, CA, [2] Southern California Kaiser Permanente Medical Group, Los Angeles, CA

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