

# Allogeneic Marrow Transplantation from HLA-Identical Related Donors for Treatment of Homozygous Thalassemia Major and other Red Cell Disease (Excluding Sickle Cell Anemia) (801)

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## Synopsis:

Hematopoietic Cell transplantation from allogeneic donors is an increasingly effective therapy for life-threatening non-malignant disorders such as aplastic anemia, Fanconi Anemia, congenital immunodeficiency disorders and inborn errors of metabolism. In addition, an evolving role for hematopoietic cell transplantation for hemoglobinopathies and red cell disorders is being defined. Improved methods of preventing graft-versus-host disease (GVHD) and infection have lowered the risk of transplant-related morbidity.

Graft failure (denoted here as rejection of the hematopoietic cell graft or recurrence of the underlying red cell disorder) can be a serious threat to otherwise successful transplantation. One approach to overcome transfusion-associated rejection has been to add antithymocyte globulin (ATG) to standard cyclophosphamide (CY) conditioning. Another approach in patients with proliferative marrows has been to add dimethylmyeleran (DMM) or busulfan (BU) to CY as additional myeloablation. In the present treatment we wish to determine the efficacy of a combined regimen of targeted level BU, CY and ATG on event-free survival following hematopoietic cell transplantation for life-threatening red cell diseases.

## Objectives

- A. To define the role and risk-benefit of hematopoietic cell transplantation in patients with life-threatening red cell diseases.
- B. To define the efficacy of the preparative regimen in ablating the genetic disease.
- C. To define the short- and long-term complications of hematopoietic cell transplantation for these diseases.