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Case Report

Successful Use of Post-Transplant Cyclophosphamide in a Hypertensive Thalassemia Major Child Undergoing Matched Sibling Donor Transplant: A Case Report

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Abstract

We describe a case of a 7 year old boy with thalassemia major who was planned for a fully matched sibling donor transplant. He was planned for GVHD prophylaxis with cyclosporine and methotrexate however, on day +1 , he developed severe hypertension. The cyclosporine was stopped and a modified PTCy regimen was used as GVHD prophylaxis. He engrafted neutrophils on day +12 and platelets on day +18. He is now 12 months post transplant and is GVHD free.

Keywords: Post-transplant cyclophosphamide; Matched sibling donor; Thalassemia; Hypertension

Abbreviations

HSCT: Haematopoietic Stem Cell Transplantation; MSD: Matched Sibling Donor; ATG: Anti-Thymocyte Globulin; NTG: Nitroglycerin; PTCy: Post-Transplant Cyclophosphamide; GVHD: Graft-Versus-Host Disease; MMF: Mycophenolate Mofetil; PTLD: Post Transplant Lymphproliferative Disease

Case Presentation

A 7 year old boy, with beta thalassemia major, diagnosed at 6 months of age, was referred to us for bone marrow transplant. He was on regular monthly blood transfusions and was on oral iron chelation with deferasirox. His hemoglobin was maintained at 8 gm/dL. Clinically, he had mild splenomegaly and his ferritin was 5000 ng/mL. He had a fully 12/12 HLA matched sister. He was thus planned for matched sibling donor hematopoietic stem cell transplantation (MSD HSCT) and was started on hypertransfusion protocol along with intravenous iron chelation with desferrioxamine. He was given two courses of immunosuppression, three weeks apart, consisting of fludarabine and dexamethasone. After completion of second course of immunosuppression, he was started on a conditioning regimen with Anti-Thymocyte Globulin (ATG), thiotepa, treosulphan, fludarabine. On day -3, intravenous cyclosporine was started. The peripheral blood stem cells from his fully matched sister, were infused on day 0. On day +1, he developed severe hypertension and was started on oral amlodipine and subsequently oral atenolol and hydralazine were added. But he continued to be severely hypertensive, hence he was started on intravenous (iv) nitroglycerin (NTG) infusion. The cyclosporine levels done at 48 hours, had not reached the therapeutic level. In view of severe refractory hypertension, most likely secondary to cyclosporine, it was stopped and a decision was made to give Post transplant cyclophosphamide (PTCy) as graft-versus-host disease (GVHD) prophylaxis at a dose of 25mg/kg/day on day +3 and day +4 along with Mycophenolate mofetil (MMF). By day + 5, his hypertension was under control and he was taken off iv NTG and by day +12, his oral anti hypertensives were stopped. Once his blood pressure stabilized, he was started on oral tacrolimus (day +20). On day +8,

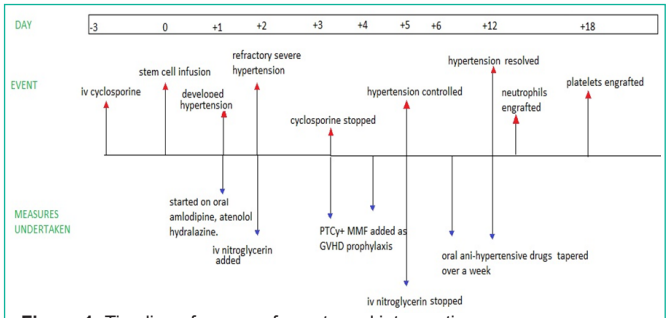

Figure 1: Timeline of course of events and interventions.

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he developed sinusoidal obstruction syndrome, which was managed with intravenous furosemide and stringent fluid management. He engrafted neutrophils on day + 12 and platelets on day +18 (Figure 1). Chimerism done on day 26 showed 100% donor chimerism. He presented 5 months after transplant with right cervical node swelling, biopsy of which revealed post transplant lymphproliferative disease (PTLD). He was treated with 4 weekly doses of rituximab as a single agent. He is currently 12 months post transplant and is doing well with no evidence of GVHD.

Discussion

To date, the only curative treatment option for thalassaemia major is allogeneic haematopoietic stem cell transplantation (HSCT) [1]. Post-transplant infections and graft-versus-host disease (GVHD) remain the major causes of morbidity and mortality in transplant recipients [2]. The combination of cyclosporine and methotrexate represents the gold standard for GVHD prophylaxis for HSCT from matched sibling donor (MSD) [3]. Hypertension is a common complication of cyclosporine and the pathogenesis is considered to be multifactorial including diminishing levels of the vasodilatory prostacyclin and endothelium derived relaxing factor [4]. In our case, we couldn't administer cyclosporine due to uncontrolled hypertension hence a modified PTCy regimen was used. There is insufficient clinical evidence for PTCy use in pediatric patients with non-malignant hematological disease [2].

Gaurav Kharya et al [5] demonstrated that PTCy can be safely used in matched unrelated donor transplant in 3 children with thalassemia major and one child with sickle cell disease. The standard-dose PTCy regimen significantly increases the risk of post transplant cytomegalovirus and BK infection and hence the concern for its use in non-malignant diseases. In a study by Lu J et at, it has been reported that low-dose PTCy (25mg/kg×2d)/low dose ATG (1.5mg/kg×3d) can reduce the risk of GVHD in matched unrelated donor transplants along with a significant reduction in viral reactivation [2]. We used low dose PTCy with MMF and tacrolimus was added later on. PTLD in our case, is probably a consequence of Epstein-Barr Virus reactivation but was managed with no complications. We conclude that randomized controlled trials will be needed for use of PTCy in matched related donor transplantation in children with benign hematological diseases to assess its safety profile and efficacy.

Ethical Approval

According to our Institutional Ethics Committee, ethical approval is not required for single case reports. However, written informed consent was obtained from the patient's parents/legal guardians.

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