



Clinical Audit

From lifeline to new life: Bone marrow transplantation in children with thalassemia major in Kauvery Hospital, Trichy

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Abstract

Background: Clinical data from Kauvery Hospital, Trichy, highlights the success of their pediatric Bone Marrow Transplantation (BMT) program for Thalassemia Major, showcasing its evolution from a "lifeline" of chronic transfusions to a "new life" of disease-free survival.

Key words: Thalassemia major; Cyclosporine; BMT

1. Introduction

Thalassemia major is one of the most common hemoglobinopathies all over the world and is the most common single gene disorder in India [1]. Improvement in transfusion and chelation techniques has improved the life span of patients with thalassemia major, however, the quality of life remains low [1]. Bone marrow transplantation (BMT) is the only realistic option available to cure thalassemia major. Treosulfan based conditioning regimen is found to be safe and effective for children with thalassemia major undergoing BMT [2].

2. Patients and methods

We performed a retrospective study on children who underwent BMT for thalassemia major in Kauvery Hospital, Trichy over a period of 3 years (2023 - 2025).

3. Results

A total of 5 children with thalassemia major underwent BMT during the study period.

4. Conditioning regimen

The conditioning regimen for all matched sibling donor transplant includes:

- Inj Thiotepa 4 mg/kg/dose IV q 12 hourly x 2 doses on day - 7
- Inj Fludarabine 40 mg/m² IV once daily for 4 days (day -6 to -3)
- Inj Treosulfan 14 g/m² IV once daily for 3 days (days -6 to -4).

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Child who underwent BMT from unrelated donor received additional rabbit ATG (Thymoglobulin) 1.5 mg/kg/day for 3 days (days -3 to -1).

5. Other supportive care measures

All patients received Cyclosporine (starting on day -2) and Methotrexate (on days +1, +3, +6, +11) as GVHD prophylaxis. Inj Caspofungin was given as antifungal prophylaxis, which was shifted to oral fluconazole or voriconazole after engraftment. Acyclovir was given as antiviral prophylaxis. Ursodeoxycholic acid was given as prophylaxis for sinusoidal obstruction syndrome. Cotrimoxazole was started from day +30 as prophylaxis against *Pneumocystis jiroveci*.

The characteristics and outcome of the patients are summarised in table 1.

Table 1

Characteristics	case 1	case 2	case 3	case 4	case 5
Age at transplant	3 years	1 year 7 months	1 year 11 months	8 years	3 years 6 months
Gender	Female	Female	Female	Female	Female
Pesaro class	class I	class I	class I	class II	class I
Donor type	Matched sibling (brother)	Matched sibling (sister)	Matched unrelated donor (male)	Matched sibling (sister)	Matched sibling (non-identical twin sister)
Donor age	11 years	9 years	26 years	2 years 6 months	3 years 6 months
HLA match	10/10	10/10	11/12 (with DP1 permissive mismatch)	10/10	10/10
Stem cell source	Peripheral blood stem cells	Peripheral blood stem cells	Peripheral blood stem cells	Peripheral blood stem cells + cryopreserved umbilical cord stem cells	Peripheral blood stem cells
CD34+ stem cell dose	7 million / kg	7 million / kg	7 million / kg	12 million/kg	7.5 million/kg

Characteristics	case 1	case 2	case 3	case 4	case 5
Date of transplant	03/06/2023	14/12/2023	08/05/2025	16/06/2025	14/10/2025
Post BMT complications	Febrile neutropenia, mucositis	Febrile neutropenia	Febrile neutropenia, mucositis	Febrile neutropenia, mucositis, Sinusoidal obstruction syndrome (resolved)	Febrile neutropenia (Ralstonia sepsis), mucositis, Sinusoidal obstruction syndrome (resolved)
Neutrophil engraftment	Day 13	Day 11	Day 11	Day 13	Day 13
Platelet engraftment	Day 13	Day 13	Day 13	Day 13	Day 13
Acute GVHD	No	Yes (grade I skin)	No	No	No
Chronic GVHD	No	No	No	No	No
CMV reactivation	No	No	No	Yes	No
Post BMT follow up (till date)	2 years 6 months	2 years	7 months	6 months	2 1/2 months
Current status	transfusion free & asymptomatic	transfusion free & asymptomatic	transfusion free & asymptomatic	transfusion free & asymptomatic	transfusion free & asymptomatic
Recent Chimerism status	100% donor	100% donor	100% donor	100% donor	100% donor

6. Discussion

Allogenic BMT remains the only curative treatment option for children with thalassemia major [3]. Availability of matched sibling donor gives the best chances of success in BMT, however alternate donor transplants also show increasing success rate with refinement of techniques in BMT. Identifying the ideal donor and performing BMT at a younger age before the cardiac and hepatic complications of iron overload sets in provides better survival rates. Our experience of 5 children with thalassemia major successfully undergoing BMT in a tier-2 city in Tamil Nadu is encouraging.

References

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