

# OUTCOME OF HAEMATOPOIETIC STEM CELL TRANSPLANTATION FOR PRIMARY IMMUNE DEFICIENCY DISORDERS IN A TERTIARY CARE HOSPITAL

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## **INTRODUCTION**

Primary immunodeficiency disorders are inherited disorders with impaired and dysregulated immunity characterised by recurrent infections, failure to thrive. Haematopoietic stem cell transplantation (HSCT) is a curative option available for many primary immune deficiency disorders (PID). In the recent years increased awareness, availability of diagnostics based on flow cytometry, genetic testing, improved supportive care, use of reduced toxicity conditioning and alternate donor HSCT have improved access to HSCT for children with PID in India. We present results on children with PID who underwent HSCT in our Hospital and the factors that influenced outcome.

### **METHODS**

This prospective observational study was conducted to know the outcome of HSCT for PID in a tertiary care hospital during the period from August 2023 to September 2024. We analysed the impact of the type of PID, conditioning regimen, Type of HSCT, cause of Mortality and overall survival

- Name	Age	Sex	Diagnosi	Outcome
		- COA	Severe	0 0 0 0 0 1 1 0
			Combine d	
			Immuno	
Mithran	1	Mch	deficienc	Good
IVIILIII aii	<b>T</b>	IVICII	y Severe	Good
			Combine	
			d	
			Immuno deficienc	
Dharshini	9	Fch	у	Good
			Chediak-	
			Higashi syndrom	
			e with	
Paruthivel			primary	
an	1	Mch	HLH Griscelli	Good
			syndrom	
Ujaifa	13	Female	е	Died
			Severe	
			Combine d	
			Immuno	
			deficienc	
	5mont		y/Omen syndrom	
Thulasi	hs	Fch	e	Died

#### RESULTS

A total of 5 children (3 female and 2 male) underwent HSCT for PID at a median age of 12 months (range 5 months to 156 months). HSCT was done for SCID, Griscelli syndrome and Chediak-Higashi syndrome. Matched family donor was available for 2 children. 1 child was transplanted with Haplo-identical donor HSCT and 2 children were transplanted with Matched Unrelated donor HSCT. Busulfan based conditioning regimen was used for 3 children and Treosulfan based conditioning regimen was used for 2 children. The graft source used was peripheral blood stem cells. The survival was superior in children receiving HSCT from Matched Unrelated donor (40%, n=2). Infection is the main cause of mortality in 2 children. The 1year overall survival rate was 60%.

# **CONCLUSION**

Matched Unrelated donor HSCT is now feasible and has made a therapeutic option accessible to all children with PID.

#### **REFERENCE:**

Gupta S, Madkaikar M, Singh S, Sehgal S, Primary Immunodeficiencies in India: a perspective, Ann N Y Acad Sci(2012) 1250:73-9.