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

AUTHORS : DR. S. SUMATHIRA, PROF : DR. B. LATHA, DR. ARUNA RAJENDIRAN, DR. G. KAVITHA

AFFILIATION : MADRAS MEDICAL COLLEGE, CHENNAI, TAMIL NADU.

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	Diagnosis	Outcome
Mithran	1	Mch	Severe Combined Immunodeficiency	Good
Dharshini	9	Fch	Severe Combined Immunodeficiency	Good
Paruthivelan	1	Mch	Chediak-Higashi syndrome with primary HLH	Good
Ujaifa	13	Female	Griscelli syndrome	Died
Thulasi	5months	Fch	Severe Combined Immunodeficiency/Omen syndrome	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.