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Haematopoietic Stem Cell Transplantation in Primary Immunodeficiencies in Lithuania

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Background. Primary immunodeficiencies (PIDs) are heterogeneous group of inborn errors of immunity. The most severe forms are life-threatening and require haematopoietic stem cell transplantation (HSCT) for curative effect. The aim of our study was to evaluate the outcomes of HSCT performed in children with PIDs in Centre for Paediatric Oncology and Haematology, Vilnius University Hospital Santaros Klinikos during 2010-2021 year period.

Methods. Medical records were retrieved and a retrospective analysis was carried out.

Results. Since 2010, when the first HSCT was performed for this patient group in Lithuania, 16 children (11 males and 5 females) underwent HSCT due to these conditions: severe combined immune deficiencies (n = 9), Wiskott-Aldrich syndrome (n = 1), chronic granulomatous disease (n = 2), haemophagocytic lymphohistiocytosis (n = 3), immune deficiency of unknown genetic variant (n = 1). Median age of the patients was 1.11 years (95% IQR 0.60-7.11). In total, five (31%) were transplanted from matched sibling donor (MSD) and 11 (69%) from matched unrelated donor (MUD). Median duration of follow-up after HSCT was 4.15 years (95% CI [3.27, 6.37]). At 3 years overall survival according to the donor type MSD vs MUD was 1.000 (95% CI [1.000, 1.000]) vs 0.623 (95% CI [0. 0.389, 0.999]), p = 0.1366, event free survival was 1.000 (95% CI [1.000, 1.000]) vs 0.455 (95% CI [0.238, 0.868]), p = 0.0558.

Conclusions. The outcomes of HSCT in children diagnosed with PIDs in our centre during 2010-2021 years period are encouraging and correspond to the results reported by main HSCT centres. The best outcomes were seen in children transplanted from a MSD.