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Outcomes of Pediatric Hematopoietic Stem Cell Transplantation in Lithuania

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Background. The pediatric hematopoietic stem cell transplantation (HSCT) program at VUHSK was launched in 2002. It provides autologous and allogeneic services for all Lithuanian and, since 2011, Latvian children.

Aims. We aimed to assess change in HSCT outcomes over two decades.

Methods. A retrospective analysis of all consecutive HSCTs from 2002 to 2021 was performed. Two time periods (2002-2011 and 2012-2021) were compared to evaluate the change. The 5-year overall survival (OS_{5y}), the cumulative incidence of relapse (CIR) in the malignant setting and transplant-related mortality (TRM) were analyzed. Descriptive statistics and Kaplan-Meier survival estimates were calculated.

Results. From 2002 to 2021 totally 269 HSCTs (56.5% (152/269) allogeneic and 43.5% (117/269) autologous) were performed in 246 unique recipients. The median annual number of allogeneic HSCT increased from 6 in 2002-2011 to 11 in 2012-2021. Non-Lithuanian citizens comprised 22.2% of all unique recipients (32/144) in 2012-2021.

In the autologous setting, no changes occurred comparing OS_{5y}, CIR and TRM in 2002-2011 (n=52/103) vs 2012-2021 (n=51/103).

In the allogeneic setting a significant improvement in OS_{5y} from 0.380 (95% CI 0.267, 0.541) in 2002-2011 (n=50/144) to 0.768 (95% CI 0.686, 0.861) in 2012-2021 (n=93/144), $p < 0.0001$, was observed. The TRM at 100 days decreased from 0.260 (95% CI 0.143, 0.380) in 2002-2011 to 0.099 (95% CI 0.046, 0.172) in 2012-2021, $p < 0.0001$ and at 5 years – from 0.507 (95% CI 0.353, 0.643) to 0.165 (95% CI 0.097, 0.249), $p < 0.0001$, respectively. None of the patients transplanted from an HLA-identical sibling in 2012-2021 (n=25) succumbed due to TRM.

The subgroup analysis of 85 patients transplanted for malignant diseases showed a significant decrease in the CIR_{5y} from 0.435 (95% CI 0.213, 0.639) in 2002-2011 (n=34/85) to 0.156 (95% CI 0.062, 0.290) in 2012-2021 (n=51/85), $p = 0.043$.

Conclusions. Despite a small transplant volume, a significant improvement over two decades was demonstrated. A close collaboration between Lithuania and Latvia and centralized patient referral is essential to maintain sufficient transplant volume and ensure safe transplant quality.