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Allogeneic HSCT for Pediatric Acute Myeloid Leukemia in Lithuania

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Background. Allogeneic hematopoietic stem cell transplantation (aHSCT) is the only curative treatment for childhood high-risk, primary refractory, or relapsed acute myeloid leukemia (AML). In Lithuania AML-BFM protocol was used to treat pediatric AML in 2002-2013. Since 2014 the first-line treatment switched to the NOPHO-DBH AML protocol. The risk stratification, indications for aHSCT differed between the protocols.

Aim. To evaluate aHSCT outcomes in children transplanted for AML at our institution in 2002-2021.

Methods. A retrospective analysis of medical records was performed. The treatment outcomes were compared between 2002-2011 (1st period) and 2012-2021 (2nd period).

Results. AML was diagnosed in 81 children in 2002-2021 (52 cases in 1st period, 29 cases in 2nd period). In the 1st and 2nd periods, out of all AML patients, 17.3% (n = 9) and 58.6% (n = 17; including 4 Latvian children) were treated with an aHSCT, respectively. Analysis of aHSCT outcomes revealed no difference in transplant-related mortality (TRM) between the 1st and the 2nd periods: the probability of TRM at 100 days was 0.220 (95 %CI [0.028, 0.530]) vs 0.120 (95 %CI [0.018, 0.320]), and at 5 years 0.615 (95% CI [0.280, 0.831]) vs 0.087 (95% CI [0.014, 0.246]), respectively (p = 0.1732). Outcomes evaluated at 5 years after aHSCT showed a statistically significant improvement from 1st to 2nd periods: the cumulative incidence of relapse decreased from 0.486 (95% CI [0.073, 0.820]) to 0.067 (95% CI [0.004, 0.269]), p = 0.0402; the probability of event-free survival increased from 0.333 (95% CI [0.132, 0.840]) to 0.765 (95% CI [0.587, 0.995]), p = 0.0462, as the probability of overall survival did: 0.333 (95% CI [0.132, 0.840]) vs 0.765 (95% CI [0.587, 0.995]), p = 0.0488, respectively.

Conclusions. The most analysed outcomes of aHSCT performed for pediatric AML improved significantly over two decades, except for early TRM. The results should be interpreted with caution due to the small sample size.