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abstract

Allogeneic hematopoietic stem cell transplantation for pediatric acute leukemia: single center experience in 150 pediatric patients transplanted in 2021-2023

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Allogeneic hematopoietic stem cell transplantation for pediatric acute leukemia: single center experience in 150 pediatric patients transplanted in 2021-2023

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Introduction: Allogeneic hematopoietic stem cell transplantation (aHSCT) is an effective and safe option for treatment of pediatric acute leukemia. Key factors for successful aHSCT in children are the combination of transplant technologies and supportive care.

Methodology: This is a retrospective analysis of 150 pediatric aHSCT for acute leukemia in Nikolay Blokhin National Medical Research Center of Oncology in 2021-2023. We aimed to define the type and cumulative incidence of complications after aHSCT for acute leukemia, as well as the overall outcomes: overall (OS) and event-free (EFS) survival.

Results: Data were received from 145 children (median of age 8.5 years, range 0.2-17) who received 150 aHSCT. Media follow-up was 23.5 months (IQR 11-47.2). Indications included acute lymphoblastic leukemia (ALL, 66.6%), acute myeloid leukemia (AML, 30.1%), and mixed-phenotype acute leukemia (MPAL, 3.3%). Conditioning regimens: ALL – TBI 12 Gy+VP+Flu, AML/MPAL: Treo+Thio+Flu. Donors: Haploidentical (haplo) – 43.7%, match related donor (MRD) –30.2%, matched unrelated donor (MUD) – 26.1%. TCR a/b/CD19 depletion was made in 56.5% of haplo, other aHSCT– PtCy.

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Stem cell source: PBSC 70.5%, BM – 28.8%, BM + PBSC – 0.7%. IST therapy: haplo with TCR a/b/CD19 depletion: tocilizumab+abatacept, PtCy– CsA/ruxolotinib, abatacept+vedolizumab, MRD - CsA/ruxolotinib, abatacept, MUD tacro/ruxolotinib, ATG, abatacept. Median of ANC recovery was 13.6 (10.3-22.6). At 3 months, the cumulative incidence for transplant related toxicities (infections, organ toxicity) of 1-2 gr. was 78% (95% Confidence Interval (CI): 59.2-96.4), 3-4 gr. was only 8.2% (95% CI: 5.1-12.8). Cumulative incidence for acute GvHD (aGvHD) of 1-2 gr. was 47.3% (95% Confidence Interval (CI): 39.6-53.2), 3-4 gr. was only 3.5% (95% CI: 2.2 6.9). Chronic GvHD (cGvHD) was found in 32 patients, 10 – extensive form. Overall, at 3-years OS was 82.1% (95% CI: 78.4-85.4) for ALL and 75.1% for AML/MPAL (95% CI: 73.3-78.7). EFS was 78.5% (95% CI: 75.8 79.9) for ALL and 69.6% for AML/MPAL (95% CI: 65.5-71.8). Nonrelapse mortality (NRM) 11.4% (95% CI: 9.6-13.5).

Conclusion: Our study confirms that aHSCT for pediatric acute leukemia is a safe and effective option. Combination of TBI-based myeloablative conditioning for ALL and pharmacological – for AML/MPAL is an effective strategy.