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abstract

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Tandem autologous hematopoietic stem cell transplantation is safe and effective for treatment of pediatric solid tumors: results of multicentre retrospective study in Russia

Author: *K. Kirgizov [1], D. Smirnova, I. Kazantsev, S. Kuleva, P. Tolkunova, Yu. Dinikina ,SmirnovaA.Yu., Lapaeva S.I. T. Aliev, I.Kostareva, K. Sergeenko, N. Stepanyan, G. Bronin, M. Natrusova, E. Machneva, E. Skorobogatova, D. Shevtsov, P.Naymushina, D.Kachanov, L. Vakhonina, L. Fechina, I. Spichak, Grachev N. S. Varfolomeeva, L. Zubarovskaya*

Affiliation: *[1] LevDurnov Research Institute of Pediatric Oncology and Hematology of N.N. Blokhin National Medical Research Centre of Oncology (Moscow, Russian Federation)*

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Introduction: Tandem autologous hematopoietic stem cell transplantation (aHSCT) is an integral part of modern approach to the treatment of pediatric high risk solid tumors. This confirms the actuality of multicenter studies of tandem aHSCT safery profile and efficacy.

Methodology: This is a retrospective multicenter study analyzing comprehensive data from the Russian pediatric transplant centers requested in a specifically self-designed questionnaire. We aimed to determine the type and cumulative incidence of complications after tandem aHSCT for pediatric solid tumors, as well as the overall outcomes: overall (OS) and event-free (EFS) survival.

Results: 107 pediatric patients (median of age 2 years, range 0.9-17) received tandem aHSCT for solid tumor between 2004 and 2024 in 9 Russian transplant centers. Median follow-up was 34.1 months (IQR 7.5-178.2). Indications included CNS tumors (CNS, 65.3%), germ-cell tumors (GCT, 17.7%), neuroblastoma (NB, 12.5%), hepatoblastoma (HB, 3.6%), and sialoblastoma (SB, 0.9%). All conditioning regimens were myeloablative. M:F=1,35:1. Status before tandem aHSCT: partial response (PR,47.6%), complete remission (CR, 45.8%), stable disease (SD, 4.8%), progression (0.9%), and mixed response (MR, 0.9%). Median of CD34+ cell dose was $5{,}45\times10^6$/kg (3.5 – 9.6) and $5{,}73\times10^6$/kg (4.1 – 10.2) for the first and second aHSCT retrospectively. There were no significant correlation between the number of CD34+ cells and the day of ANC recovery.

Stem cell source: PBSC – 93.4%, BM – 4.8%, BM + PBSC – 1.8%. Median of ANC recovery was 11.2 (9.5-13.5) and 12.7 (10.8-16.1) days for first and second aHSCT retrospectively. At 3 months, the cumulative incidence for transplant related toxicities (infections, hepatic and nephrotoxicity) of 1-2 gr. was 81%(95% Confidence Interval (CI): 61.2-98.3), 3-4 gr. wasonly 10.2% (95% CI: 6.5-13.2).Overall, at 3-years OS was 72.2(95% CI: 69.3-76.9), and EFS was 62.7% (95% CI: 59.7-64.1).Non-relapse mortality (NRM) 4.0% (95% CI: 2.8-6.4).

Conclusion: Our study confirms that tandem aHSCT for a pediatric solid tumor is safe and effective option. Transplant-related toxicity (both infectious and non-infectious) is acceptable. NRM is comparable low. Our study highlights the importance of multicenter collaboration in pediatric solid tumors aHSCT, especially in tandem HSCT application.