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abstract

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Autologous Hematopoietic Stem Cell Transplantation for "Rare" Solid Malignant Diseases in Children. Experience of the N.N. Blokhin National Medical Research Center of Oncology

Author: *T.Z. Aliev [1], E.B. Machneva, I.O. Kostareva, K.A. Sergeenko, D.S. Smirnova, N.A. Burlaka, Yu.V. Lozovan, I.Yu. Trushkova, A.Yu. Elfimova, K.V. Mitrakov, T.I. Potemkina, M.D. Malova, R.R. Fatkhullin, N.G. Stepanyan, G.B. Sagoyan, A.M. Suleymanova, N.V. Matinyan, G.M. Muftakhova, A.P. Kazantsev, O.M. Romantsova, M.V. Rubanskaya, T.L. Ushakova, P.A. Kerimov, A.D. Rodina, Yu.V. Skvortsova, I.V. Kazantsev, A.S. Slinin, T.V. Gorbunova, P.A. Kerimov, V.G. Polyakov, K.I. Kirgizov, S.R. Varfolomeeva*

Affiliation: *[1] Lev Durnov 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: One of the key stages of therapy for children with "rare" solid malignant neoplasms (MN), improving the prognosis for the underlying disease, is autologous hematopoietic stem cell transplantation (auto-HSCT). In world practice, this therapy is carried out for children with common diseases such as malignant neoplasms (MN), however, there is a small cohort of patients with "rare" diseases, with which not all centers have experience in carrying out HSCT in children.

Methodology: At the Research Institute of Pediatric Oncology and Hematology (RI DOiG) of the N.N. Blokhin National Medical Research Center of Oncology, auto-HSCT was performed in 12 patients over 5 years (median age 7.6 years, range 1.3-17.2). The median follow-up was 37.5 months (interquartile range 5.1-78.3). Indications included retinoblastoma (9 patients, RB), pleuropulmonary blastoma (1 patient, PPB), sialoblastoma (1 patient, SB), hepatoma (1 patient, HB). M:F= 7:5. Stem cell source: PBSC – 100%. Conditioning regimens: PPB – Treo/Mel, HB – Eto/Carbo, SB/RB – thiotepa-based regimens. The median CD34+ cell dose was 5.27×10 6/kg (2.4 – 15.1).

Results: The patients successfully underwent the HSCT stage. In the early stages after HSCT, complications were noted: toxicoderma up to grade 2-3, oropharyngeal mucositis up to grade 1-3, neutropenic enterocolitis grade 1-2, infections, hepato- and nephrotoxicity. These complications were stopped. All patients engrafted. The median ANC recovery was 12 (9.7–16.2) days. No significant toxicity was recorded. There were 3 fatal outcomes in patients with RB associated with relapse of the underlying disease.

Conclusion: Due to the low frequency of occurrence of these diseases in medical practice, as well as late treatment/referral to specialized medical centers, there is no standard therapy and experience of working with these patients in the early and late stages of HSCT. Each patient with a "rare" disease requires an individual approach to management at the stage of HSCT.