HEMATOLOGY, TRANSFUSION AND CELL THERAPY

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PEDIATRIC PRESENTATIONS

Sp01

HEMATOPOIETIC STEM CELL TRANSPLANTATION IN CHILDREN WITH CENTRAL NERVOUS SYSTEM TUMORS

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Central nervous system (CNS) tumors are still the most common malignant solid tumors in childhood and constitute 16-25% of all tumors (1). Most tumor types include malignant gliomas, ependymoma,medulloblastoma, and atypical teratoid rhabdoid tumors (2). Surgery, radiotherapy (RT), and chemotherapy are the primary treatment modalities, and the prognosis in some histopathological subtypes and recurrent or residual diseases is, unfortunately, still poor. In cases under three, avoiding radiotherapy due to the long-term side effects adversely affects the prognosis. For this reason, the studies on high-dose chemotherapy with autologous hematopoietic stem cell transplantation (HDC/AuHSCT) are mostly conducted on recurrent CNS tumors, cases under three years old, and medulloblastoma which is a chemosensitive tumor.

The most crucial factor in increasing the success of the transplant is minimizing the tumor burden before transplantation. The minimal residual disease generally includes residual tumor <1.5 cm2, no tumor cells in cerebrospinal fluid, and minimal radiological signs in metastatic sites (3). Chemotherapeutics with good CNS penetration should be selected in the conditioning regimen. Carmustine, thiotepa, and melphalan are some of these drugs (4).

Considering the low number of patients with malignant gliomas, the 4-year overall survival (OS) rates range from 30-46% (5,6), while the 2-year OS rates were found to be 46% in the study of the Children Cancer Group with 86 cases. The study was terminated early due to pulmonary toxicity (7). In pontine gliomas and ependymomas, the effect of transplantation on treatment success has not been demonstrated (8-11). In a meta-analysis evaluating patients with metastatic atypical teratoid rhabdoid tumors, HDC/AuHSCT was shown to improve survival ($p<0.0001$) and reduce the risk of mortality ($p<0.0001$) (12). The study of the European Rhabdoid Registry has shown that selected cases may benefit from transplantation together with RT (13). In the study performed by the St. Jude group on newly diagnosed medulloblastoma cases, 5-year event-free survival (EFS) was found to be 83% and 70% in the high-risk and average-risk groups (14). In the Head Start III study, RT was not applied to the patients who were younger than six years of age and had nonmetastatic tumors at diagnosis, and had no residual tumors after induction therapy, and HDC/AuHSCT was performed. The 3-year RT-free EFS was 49.5% in the whole group, and the 5-year EFS was 88% in the desmoplastic group (15). The Children Oncology Group's study applied tandem consolidation treatment with HDC/AuHSCT to 36 medulloblastoma cases. Five-year EFS was 60% in the entire group (16). These studies show that a high survival rate can be achieved without affecting neurocognitive functions.

In conclusion, HDC/AuHSCT is a treatment option that can be applied in some CNS tumors. Specifically, it can be applied when the patient is under three years of age, without affecting neurocognitive functions and reducing survival rates despite not performing RT.

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STEM CELL TRANSPLANTATION AS A TREATMENT OPTION FOR RELAPSED/ REFRACTORY GERM CELL TUMORS

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Malignant germ cell tumors (GT) arise from abnormal migration of primordial germ cells and are histologically identical whether they occur inside or outside of central nervous system (CNS) (1). They are divided into two heterogenous groups: germinomas and non-germinomatous germ cell tumors according to histological findings. Although the optimal treatment strategy remains a matter of debate, they generally respond well to surgery, radiotherapy, chemotherapy, or a