

Successful outcome with tandem myeloablative chemotherapy and autologous peripheral blood stem cell transplants in a patient with atypical teratoid/rhabdoid tumor of the central nervous system

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Abstract Atypical teratoid rhabdoid tumors (ATRT) are highly malignant tumors of the central nervous system with a peak incidence in children less than 3 years of age. Despite multimodal therapy including surgery, radiation and chemotherapy, the prognosis remains dismal. No specific treatment guidelines are defined for ATRTs but a gross total resection and radiation therapy (RT) appear to improve overall outcome. In children less than 3 years of age, the prognosis is dismal due in part to the reluctance to utilize RT given its severe neurological sequelae. To avoid RT in this age group, intensification of chemotherapy has been tried and has shown to improve outcome. Myeloablative chemotherapy followed by autologous stem cell re-infusion has been used as a modality to intensify therapy but there are no reports of use of tandem myeloablative regimens and autologous stem cell re-infusions for treatment of ATRT. We herein report the case of a 4-month-old boy with ATRT with partial resection of his tumor who achieved complete remission using tandem high-dose

therapy followed by autologous peripheral blood stem cell re-infusions despite having biopsy proven disease at the time of starting the tandem regimens. This was achieved without the use of RT as a treatment modality.

Keywords Atypical teratoid rhabdoid tumor · Stem cell transplant · Radiation therapy

Introduction

Central nervous system (CNS) tumors are the second most common pediatric tumors with an overall survival of 70% [1]. Rhabdoid tumors of the CNS, also known as atypical teratoid rhabdoid tumors (ATRT), comprise of approximately 2–3% of pediatric brain tumors with a peak incidence in children less than 3 years of age [2]. They are associated with characteristic genetic abnormalities, which include either monosomy 22 or deletion involving the *hSNF5/INI1* gene located on 22q11.2 thus leading to the absence of INI1 protein which is a hallmark of ATRT [3, 4]. ATRTs are highly malignant tumors with a median survival of less than 1 year after diagnosis. Due to the small numbers and relatively recent recognition of these tumors as a distinct entity, there are no standards of treatment defined yet. Two prognostic factors that affect overall outcome are: (1) the extent of tumor resection and (2) early radiation therapy (RT) [2]. Given the young age of many patients with ATRT, the short and long term effects of RT are prohibitively severe. Therefore, chemotherapy is the treatment of choice after surgery for patients less than 3 years old. Intensification of chemotherapy appears to improve outcome [5–7]. The use of myeloablative chemotherapy followed by autologous stem cell rescue as a modality to intensify therapy has been reported

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