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The outcome of treatment in patients with high-risk (HR) retinoblastoma

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Abstract

From 2001 to 2008 16 patients (8 female, 8 male) with high-risk retinoblastoma (RB) were treated in our center. HR was defined as microscopic residual tumor after enucleation (stage II, n=8); regional extension (stage III, n=4): a) overt orbital disease (n=3) b) involvement of regional or cervical lymph nodes (n=1); metastatic disease (stage IV): a) metastasis (multiple lesions (n=1)), b) CNS extension: prechiasmatic lesion (n=3). The median age was 32 (8–115) years and the median body weight was 13.8 (9.8–32) kg. Two patients (pts) had bilateral RB (1st case: operated on left eye and relapsed in the right eye with orbital involvement 3 months after surgery; second: metastatic disease with prechiasmatic lesion). The induction phase included 4 courses of CT including cyclophosphamide, VP-16 and carboplatin, surgery and external beam RT at a dose of 50 Gy. PBSCs were harvested after the first course of CT. Conditioning included busulfan 16 mg/kg and melphalan 140 mg/m^2, followed by autologous SCT. Twelve pts received a median number of 3.5 (2.2–4.8)x10^6 CD34+ cells/kg. Toxicity was moderate in all but one pt (stage III), who died on d+8 from sepsis caused by Klebsiella pneumonia. The median number of days to WBC >1.0x10^9/l, Plt >20, and 50x10^9/l was 10 (9–11), 14 (10–20), and 17 (14–35) days respectively. Three pts died due to relapse: in CNS (n=2), and one with metastatic disease experienced a progression of disease in bone, BM, testis and lymph nodes 8 months after HDCT. Eight patients are alive and well at follow-up of 55 months. EFS and DFS were 64% and 68% respectively. Three patients refused HDCT. Two out of 3 (both with prechiasmatic lesion) relapsed in CNS and died. One with stage II is alive and disease free. One pt with overt orbital disease progressed when on treatment and later died.

Keywords: high-risk retinoblastoma, high-dose chemotherapy, autologous stem cell transplantation