Carboplatin-based primary chemotherapy for infants and young children with CNS tumors.


Cincinnati Children's Hospital Medical Center, Cincinnati, OH 45229, USA. fouladi@chmc.org

BACKGROUND: A carboplatin-based chemotherapy regimen was used as primary postoperative therapy in infants with central nervous system (CNS) tumors to limit renal and ototoxicity and to target systemic exposure. METHODS: Fifty-three patients aged <age 3 years with embryonal CNS tumor medulloblastoma (n = 20), ependymoma (EP, n = 21), choroid plexus carcinoma (CPCA, n = 5), and primitive embryonal neoplasms including atypical teratoid rhabdoid tumors (n = 7) were treated with cyclophosphamide, etoposide, and carboplatin. Radiation therapy was used only for residual disease at the end of chemotherapy or disease progression. RESULTS: The response rate after 2 cycles of chemotherapy was 34% (complete response, 13.8%; partial response, 20.7%). Myelosuppression was the dominant toxicity; 2 patients had toxic deaths related to thrombocytopenia with trauma. The 5-year overall survival (OS) was 49% +/- 7%, and the progression-free survival (PFS) was 31% +/- 7%, with a median follow-up of 11.4 years (range, 5.2-15.0 years). For medulloblastoma, the 5-year PFS was 26% +/- 9%; for EP it was 33% +/- 10%; for CPCA it was 80% +/- 18%; and for primitive neuroectodermal and atypical teratoid rhabdoid tumors it was 0%. Localized EP patients with gross total resection who did not undergo radiotherapy had a 5-year PFS of 57% +/- 17% and OS of 71% +/- 16%. Two patients developed late second malignancies; 1 was associated with germline p53 mutation. CONCLUSIONS: The results confirm that carboplatin has similar activity to cisplatin in otherwise similar regimens. Five-year survival data are comparable to those reported in other recent studies, including high-dose chemotherapy studies. Of note is the marked activity in CPCA and gross totally resected EP.