Abstracts

MDB-51. PHASE II INTERVENTIONAL STUDY TO EVALUATE THE SAFETY AND EFFICACY OF STANDARD AND HIGH-DOSE CHEMOTHERAPY COMBINED WITH CRANIOSPINAL IRRADIATION WITH PROTON THERAPY IN PATIENTS WITH METASTATIC MEDULLOBLASTOMA AND OTHER EMBRYONIC CANCERS: PRELIMINARY RESULTS

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BACKGROUND: The high risk medulloblastoma remains a leading cause of cancer-related death in children. METHODS: A total of 18 patients have been enrolled from February 2018 to May 2022. The median age was 7.5 years (range: 12 males and 6 females). The protocol predicted the first phase of induction with 4 cycles of chemotherapy. After induction patients with favorable histology without evidence of disease were enrolled to proton therapy (with concomitant vinorelbine b.iweekly). The maintenance phase with Lomustine repeated every 9 weeks and vinorelbine every 3 weeks for overall 12-18 months (PR). Conversely L2A MB, were subjected to consolidation phase with high dose of chemotherapy with thiopeta followed by autologous HSC transplant and successive maintenance phase of 6 months. RESULTS: At October 2023, the median follow-up was 24 months. After the induction phase 14 patients showed CR, 2 PR, 1PD, 1SD. At subsequent re-evaluations two with PR, showed disease control, one CR and the other SD and the patient with PD showed CR. Patient with SD showed an unchanged state of the disease. During the reporting period two patient died for progression of the underlying disease. At 24 months from the start of treatment the PFS was 75.4 % (95% CI: 34.1% - 87.6%) and at 15 months the OS was 86.1% (95% CI: 68.1% - 99.2%). The principal expected side effects of chemotherapy were hematologic toxicity and 38% rate of GH deficiency. Among the unexpected side effect was CMV retinopathy with complete blindness and leukoencephalopathy after high dose of thiopeta and proton irradiation. We reported only one case of ototoxicity. CONCLUSIONS: The use of proton therapy combined with intensive chemotherapy and myeloablative chemotherapy only in selected cases and always before irradiation has proven feasible and effective in the treatment of high-risk patients medulloblastomas and other embryonic tumors.