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Risk-adapted craniospinal radiotherapy followed by high-dose chemotherapy and stem-cell rescue in children with newly diagnosed medulloblastoma (St Jude Medulloblastoma-96): long-term results from a prospective, multicentre trial.

[Gajjar A](#), [Chintagumpala M](#), [Ashley D](#), [Kellie S](#), [Kun LE](#), [Merchant TE](#), [Woo S](#), [Wheeler G](#), [Ahern V](#), [Krasin MJ](#), [Fouladi M](#), [Broniscer A](#), [Krance R](#), [Hale GA](#), [Stewart CF](#), [Dauser R](#), [Sanford RA](#), [Fuller C](#), [Lau C](#), [Boyett JM](#), [Wallace D](#), [Gilbertson RJ](#).

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Erratum in

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Abstract

BACKGROUND: Current treatment for medulloblastoma, which includes postoperative radiotherapy and 1 year of chemotherapy, does not cure many children with high-risk disease. We aimed to investigate the effectiveness of risk-adapted radiotherapy followed by a shortened period of dose-intensive chemotherapy in children with medulloblastoma.

METHODS: After resection, patients were classified as having average-risk medulloblastoma (< or = 1.5 cm² residual tumour and no metastatic disease) or high-risk medulloblastoma (> 1.5 cm² residual disease or metastatic disease localised to neuraxis) medulloblastoma. All patients received risk-adapted craniospinal radiotherapy (23.4 Gy for average-risk disease and 36.0-39.6 Gy for high-risk disease) followed by four cycles of cyclophosphamide-based, dose-intensive chemotherapy. Patients were assessed regularly for disease status and treatment side-effects. The primary endpoint was 5-year event-free survival; we also measured overall survival. This study is registered with ClinicalTrials.gov, number NCT00003211.

FINDINGS: Of 134 children with medulloblastoma who underwent treatment (86 average-risk, 48 high-risk), 119 (89%) completed the planned protocol. No treatment-related deaths occurred. 5-year overall survival was 85% (95% CI 75-94) in patients in the average-risk group and 70% (54-84) in those in the high-risk group (p=0.04); 5-year event-free survival was 83% (73-93) and 70% (55-85), respectively (p=0.046). For the 116 patients whose histology was reviewed centrally, histological subtype correlated with 5-year event-free survival (p=0.04): 84% (74-95) for classic histology, 77% (49-100) for desmoplastic tumours, and 57% (33-80) for large-cell anaplastic tumours.

INTERPRETATION: Risk-adapted radiotherapy followed by a shortened schedule of dose-intensive chemotherapy can be used to improve the outcome of patients with high-risk medulloblastoma.

Comment in

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