

Multicenter Study *Pediatr Blood Cancer*. 2026 May;73(5):e70181.

doi: 10.1002/1545-5017.70181. Epub 2026 Mar 13.

Group 4 Medulloblastomas of Early Childhood Treated With High-Dose Chemotherapy- and Craniospinal Irradiation-Sparing Approach

Patricia Orduña^{1 2}, Rose Daynielle Cansanay¹, Craig Erker³, Erin Ratterman⁴, Mary Pat Schlosser⁵, Rebecca Ronsley⁴, Chantel Cacciotti⁶, Sylvia Cheng⁷, Juliette Hukin⁸, Dolly Aguilera⁹, Claire Mazewski⁹, Vanan Magimairajan¹⁰, Natasha Pillay-Smiley¹¹, Sameer Farouk Sait¹², Andrew Cluster¹³, Mohamed S Abdelbaki¹³, Ashley S Margol¹⁴, George Michael¹⁵, Susan Chi¹⁶, Ralph Salloum¹⁶, Virginia Harrod¹⁷, Lindsey Hoffman¹⁸, Annalise Bracher¹⁸, Akanksha Senapati¹⁹, Vijay Ramaswamy²⁰, Michal Zapotocky²¹, Vicente Santa-Maria Lopez²², Kathleen Doris²³, Martin Mynarek²⁴, Stefan Rutkowski²⁴, Alvaro Lassaletta²⁵, Eric Bouffet²⁰, Lucie Lafay-Cousin¹

PMID: 41823174 DOI: 10.1002/1545-5017.70181

Abstract

Background: Group 4 medulloblastoma (MB) is rare in young children. Data on craniospinal irradiation (CSI)-sparing approaches are limited.

Methods: This multicenter study reported outcomes for patients younger than 7 years old with Group 4 MB treated with high-dose chemotherapy (HDC) without adjuvant CSI.

Results: Thirty-eight patients were included (26 M/12 F). Median age at diagnosis was 46.4 months (25.9-78), with 24% \leq 36 months. Twenty-four patients (63.2%) had localized disease. Fourteen (36.8%) presented with metastatic disease, and 26 (68.4%) underwent gross total resection (GTR). The most used HDC regimens were carboplatin/thiotepa (76.3%) and carboplatin/thiotepa/etoposide (21.1%). Twenty (52.6%) relapses occurred at a median 21.9 months (5-99.8) from diagnosis. Patients with upfront GTR and/or who received three cycles of HDC had better PFS ($p = 0.02$ and $p = 0.002$, respectively). Local relapse accounted for 45%. Eighteen patients received salvage therapy with curative intent, all with radiotherapy (16 = CSI, 2 = focal). The CSI dose ranged from 18 to 36 Gy, and 43.7% received \leq 23.4 Gy. Patients who underwent salvage surgery and/or chemotherapy were more likely to receive CSI \leq 23.4 Gy ($p = 0.008$ and $p = 0.03$). The 5-year post-relapse survival and overall survival (OS) were, respectively, 60.3% (95% confidence interval [CI]: 26.9-82) and 72.7% (95% CI: 51.9-85.7). The 5-year CSI-free OS was 69.7% (95% CI: 39.1-87.1).

Conclusion: High dose chemotherapy for Group 4 MB was associated with a relapse rate of 52.6%, which favorably compares to data reported with conventional chemotherapy. Salvage radiotherapy retrieved more than two-thirds of the patients. Half of the survivors never received radiotherapy. Such an approach may represent an alternative to upfront CSI for young children with Group 4 MB.

Keywords: craniospinal irradiation avoidance; high-dose chemotherapy; infant medulloblastoma.