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High-dose chemotherapy with autologous stem cell rescue for children with recurrent malignant brain tumors.

Shih CS, Hale GA, Gronewold L, Tong X, Laningham FH, Gilger EA, Srivastava DK, Kun LE, Gajjar A, Fouladi M

Cancer 2008 Mar 15 **112**(6):1345-53 [abstract on PubMed] [citations on Google Scholar] [related articles] [full text] [order article]

Selected by | Eric Bouffet
Evaluated 14 Mar 2008
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Study population > Humans | Child (6-12 yrs) | Adolescent (13-18 yrs)
Article type > Observational study

Faculty Comments & Author Responses

Faculty Member	Comments
Eric Bouffet The Hospital For Sick Children, Canada ONCOLOGY Controversial	**This study challenges the role of high-dose chemotherapy (HDC) in the management of recurrent pediatric central nervous system (CNS) tumors. High-dose chemotherapy is no more a standard option for children with recurrent or refractory CNS tumors. Indications for HDC are highly questionable and should be revisited at a national/international level.** HDC has been used since 1990 as a salvage treatment in children with recurrent and refractory CNS tumors. Although some series have reported encouraging results, issues regarding potential selection bias have been raised and reproducibility of these results has been questioned. This report from St. Jude Children's Research Hospital describes the outcome of 27 children with recurrent or refractory CNS tumor (medulloblastoma, supratentorial primitive neuroectodermal tumor, pineoblastoma, atypical rhabdoid/teratoid tumor, ependymoma and high-grade glioma) treated with HDC. Whenever possible, tumor burden was reduced surgically or with conventional chemotherapy prior to HDC, and nine patients had no evidence of disease at the time of the procedure. Twenty-two patients (81%) experienced disease recurrence at a median of four months after HDC and only one patient among this group was alive at the time of the publication. Among six survivors, five received both HDC and radiotherapy (including craniospinal radiation in three patients) as part of their salvage treatment. Four long-term survivors were aged <3 years at diagnosis and had received chemotherapy only as part of their frontline therapy. The authors conclude that their data do not support the use of HDC in this population and that the better outcome

observed in younger patients is most likely related to the use of radiation as part of their salvage treatment. By contrast, older children who have received multimodality therapy, including radiotherapy as part of their frontline treatment regimen, and subsequently experience disease recurrence, do not benefit from HDC alone as salvage therapy. They advocate a careful reassessment of current guidelines for these patients and the development of new strategies to improve their outcome. This study confirms data from non-selected observations and points out the likely bias of previous promising publications on HDC in this population.

Competing interests: No potential interests relevant to this article were reported.

Evaluated 14 Mar 2008

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Faculty of 1000 Medicine: evaluations for Shih CS et al *Cancer* 2008 Mar 15 112 (6) :1345-53 http://www.f1000medicine.com/article/id/1104137/evaluation

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