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Treatment of medulloblastoma with hedgehog pathway inhibitor GDC-0449.

Rudin CM, Hann CL, Laterra J, Yauch RL, Callahan CA, Fu L, Holcomb T, Stinson J, Gould SE, Coleman B, LoRusso PM, Von Hoff DD, de Sauvage FJ, Low JA.

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Comment in:

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Medulloblastoma is the most common malignant brain tumor in children. Aberrant activation of the hedgehog signaling pathway is strongly implicated in the development of some cases of medulloblastoma. A 26-year-old man with metastatic medulloblastoma that was refractory to multiple therapies was treated with a novel hedgehog pathway inhibitor, GDC-0449; treatment resulted in rapid (although transient) regression of the tumor and reduction of symptoms. Molecular analyses of tumor specimens obtained before treatment suggested that there was activation of the hedgehog pathway, with loss of heterozygosity and somatic mutation of the gene encoding patched homologue 1 (PTCH1), a key negative regulator of hedgehog signaling. 2009 Massachusetts Medical Society

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