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Long-term Efficacy of Single-agent Vemurafenib for Pleomorphic Xanthoastrocytoma.

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Pleomorphic xanthoastrocytoma is a malignant brain tumor that has a good prognosis with complete resection but does not respond well to chemotherapy if there is residual tumor. BRAFV600E mutations are common in pleomorphic xanthoastrocytomas and provide an additional means for treatment when excision is not possible. Monotherapy with the BRAF V600E inhibitor vemurafenib has only been reported in a small number of cases and mostly in adults. We present the case of a 16-year-old male who responded to vemurafenib monotherapy initially and had an additional response to vemurafenib following progression after a brief time off the medication.

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