

PubMed

Format: Abstract

Full text links

J Neurosurg Pediatr. 2016 Dec 23:1-6. doi: 10.3171/2016.9.PEDS16328. [Epub ahead of print]



## Report of effective trametinib therapy in 2 children with progressive hypothalamic optic pathway pilocytic astrocytoma: documentation of volumetric response.

Miller C<sup>1</sup>, Guillaume D<sup>1</sup>, Dusenbery K<sup>2</sup>, Clark HB<sup>3</sup>, Moertel C<sup>4</sup>.

### Author information

### Abstract

Brain tumors are the most common solid tumor in childhood, and astrocytomas account for the largest proportion of these tumors. Increasing sophistication in genetic testing has allowed for the detection of specific mutations within tumor subtypes that may represent targets for individualized tumor treatment. The mitogen-activating protein kinase (MAPK) pathway and, more specifically, BRAF mutations have been shown to be prevalent in pediatric pilocytic astrocytomas and may represent one such area to target. Herein, the authors describe 2 cases of inoperable, chemotherapy-resistant pediatric pilocytic astrocytomas with a documented response to trametinib, an MAPK pathway inhibitor. While these cases were not treated in the setting of a clinical trial, their data support further ongoing clinical trial investigation to evaluate the safety and efficacy of this agent in pediatric low-grade gliomas.

**KEYWORDS:** BRAF mutation; CNS = central nervous system; MAPK = mitogen-activating protein kinase; MAPK pathway; astrocytoma; oncology; pediatric; trametinib

PMID: 28009226 DOI: [10.3171/2016.9.PEDS16328](https://doi.org/10.3171/2016.9.PEDS16328)

[PubMed - as supplied by publisher]



LinkOut - more resources

PubMed Commons

[PubMed Commons home](#)

0 comments

[How to join PubMed Commons](#)