Case Reports Neurooncol Pract. 2025 Dec 31;13(2):428-431. doi: 10.1093/nop/npaf131. eCollection 2026 Apr.

First report of a clinical and radiologic response to vorasidenib in a patient with isocitrate dehydrogenase-mutant brainstem astrocytoma

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PMID: 42111031 PMCID: PMC13153679 (available on 2026-12-31) DOI: 10.1093/nop/npaf131

Abstract

Background: IDH (isocitrate dehydrogenase)-mutant gliomas of the brainstem are exceedingly rare. In the INDIGO trial, patients with brainstem gliomas were excluded, thus, no data exists on the use of vorasidenib in this group.

Case summary: We describe the case of a 20-year-old female who developed progressive left facial myokymia and binocular horizontal diplopia on leftward gaze over two years. Brain MRI demonstrated a non-enhancing, expansile T2/FLAIR hyperintense lesion in the left greater than right brainstem extending from the dorsal pons into the cervicomedullary junction. The slowly progressive nature of her symptoms and radiologic appearance were most suggestive of a lower-grade, infiltrative neoplasm. She underwent biopsy of the left middle cerebellar peduncle, which confirmed an IDH R132C-mutant astrocytoma, WHO grade 2. With shared decision making, the patient elected to initiate therapy with vorasidenib 40 mg once daily. After six months of treatment, her diplopia and facial myokymia nearly resolved. By eleven months, her symptoms remain well controlled. Brain MRI shows significant reduction in the size of the T2/FLAIR hyperintense lesion.

Conclusions: This case is the first to report a favorable clinical and radiologic response to vorasidenib in a patient with an IDH-mutant brainstem astrocytoma, suggesting the potential use of IDH inhibitors in this group.

Keywords: IDH inhibitors; IDH-mutant gliomas; brainstem gliomas; targeted therapy; vorasidenib.