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FDA highlights pediatric brain tumor treatment advances

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Pediatric brain tumors are a leading cause of cancer-related death in children and can lead to substantial disease and treatment-related morbidities. The development of new therapies has historically been challenging for many reasons, including disease rarity and generally poor central nervous system penetrance of small and large molecules due to the blood-brain barrier.

Recent advances in the development of targeted therapies have led to new treatment options for patients with brain tumors with certain molecular alterations.

The Food and Drug Administration (FDA) has approved several new treatments for pediatric brain tumors in recent years, marking important advances in care for children with these conditions facing an unmet medical need.

In August 2025, FDA granted accelerated approval to dordaviprone, a protease activator, for adult and pediatric patients 1 year and older with diffuse midline glioma (DMG) harboring an H3K27M mutation with progressive disease following prior therapy. This was the first FDA approval of a systemic therapy for H3K27M-mutant DMG, a particularly aggressive brain tumor that includes most patients with diffuse intrinsic pontine glioma (DIPG). The approval was based on overall response rate and duration of response among 50 patients with H3K27M DMG who had progressed after prior therapy. Additional studies will be conducted as a post-marketing requirement to verify clinical benefit.

Other recent approvals for pediatric patients with brain tumors include:

- Tovorafenib is a kinase inhibitor indicated for patients 6 months and older with relapsed or refractory pediatric low-grade glioma harboring a BRAF fusion or rearrangement, or BRAF V600 mutation (accelerated approval, April 2024).
- Vorasidenib is an isocitrate dehydrogenase-1 (IDH1) and IDH2 inhibitor indicated for patients 12 years and older with Grade 2 astrocytoma or oligodendroglioma with a susceptible IDH1 or IDH2 mutation following surgery (traditional approval, August 2024).

- Dabrafenib with trametinib, both kinase inhibitors are indicated for pediatric patients 1 year and older with low-grade glioma (LGG) with a BRAF V600E mutation who require systemic therapy (traditional approval, March 2023). This combination also is approved as a treatment for adult and pediatric patients with unresectable or metastatic solid tumors with BRAF V600E mutation whose cancer has progressed following prior treatment and who have no satisfactory alternative treatment options (accelerated approval, June 2022); supporting this approval are data from pediatric patients with high- and low-grade glioma with BRAF V600E mutations.

These advances build on decades of laboratory, translational and clinical research supported or incentivized by federal health agencies, including the FDA and the National Cancer Institute, in partnership with academic leaders, patient advocates, international regulators and industry colleagues. In 2025, the FDA held a public workshop involving advocates, investigators, regulators and industry focused on developing registries in oncology, with a specific focus on efforts in the development of registries for DMG/DIPG. The workshop provided the first step in a longitudinal effort to optimize registry development in DMG/DIPG and encourage the collection of fit-for-use high quality data that potentially could support the development of novel therapeutics for patients with DMG/DIPG.

The FDA's Oncology Center of Excellence, Office of Pediatric Therapeutics, and Office of New Drugs' Office of Oncologic Diseases and Division of Pediatrics and Maternal Health contributed to this article.

Resource

- Information on FDA's Future of Registries in Oncology Public Workshop