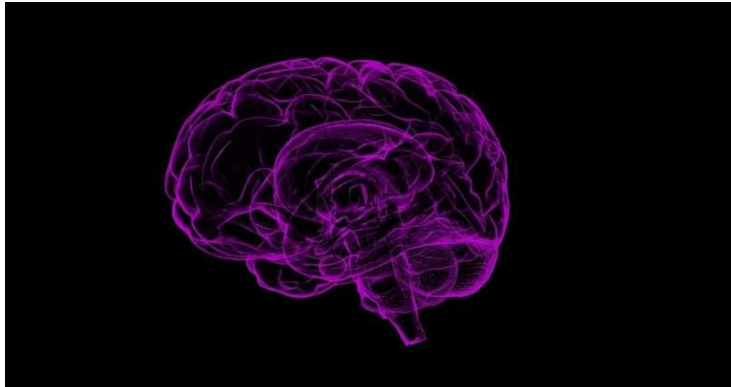


New research aims to improve treatment for pediatric brain cancer

by [Virginia Tech](#)



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Virginia Tech researchers are developing more precise treatments for pediatric brain cancer using a new class of therapies that target cancer cells while sparing healthy tissue.

Pediatric [glioblastoma](#) is rare but devastating, often leading to rapid progression, limited treatment options, and poor survival rates. Survivors may face long-term neurological and developmental challenges because of the tumor and the toxicity of current treatments.

"Our goal is to develop therapies that are both more precise and more effective for children with this devastating disease," said Kathleen Mulvaney, a cancer biologist with the Fralin Biomedical Research Institute Cancer Research Center in Washington, D.C. "We're combining cutting-edge science with new drug delivery techniques to see if we can make a meaningful difference."

Mulvaney presented her research at the [CCF Research Symposium](#).

Mulvaney aims to understand how cancer cells grow and survive using advanced tools in functional genomics and molecular analysis. Her team is focusing on a protein called KAT6A, which plays a role in these cancer-driving pathways.

While some experimental drugs exist that target both KAT6A and a related protein, KAT6B, there are currently no drugs that focus on KAT6A alone. This study will test whether a more targeted approach using KAT6A-selective compounds could be more effective against pediatric glioblastoma.

The team also plans to explore ways to deliver these compounds more efficiently to brain tumors.

Low-intensity focused ultrasound

One approach will involve low-intensity focused ultrasound—a non-invasive technique available through the Fralin Biomedical Research Institute—to help deliver therapeutics

through the blood-brain barrier, a layer of tightly connected endothelial cells that protects the brain but also prevents many potentially beneficial drugs from reaching tumors.

The team will test this method using Pf9363, an experimental compound that selectively targets KAT6A. By using focused ultrasound to temporarily open the blood-brain barrier, the researchers aim to deliver Pf9363 more effectively to [tumor](#) sites and evaluate its potential in treating pediatric glioblastoma.

The one-year study could lead to better treatments for nearly half of children with glioblastoma, whose tumors carry specific genetic changes. It may also provide the early evidence needed to support future clinical trials combining multiple therapies.

Provided by Virginia Tech

-medicalxpress.com, June 4, 2025