

Dual therapy shows promise for childhood brain cancer

by [University of New South Wales](#)



Credit: Children's Cancer Institute

Researchers at the Children's Cancer Institute and UNSW Sydney in Australia have tested a new way of treating childhood brain cancer by combining two medicines in lab studies. They found using the two treatments together may work better than using either on its own.

The research is published in the journal *Science Translational Medicine*.

In the new study, Children's Cancer Institute and UNSW Sydney researchers lab-tested a combined therapy approach on a group of difficult-to-treat brain tumors: diffuse midline gliomas (DMG). This group includes diffuse intrinsic pontine glioma (DIPG), a rare but fatal childhood brain cancer. Children diagnosed with DIPG usually survive for about 12 months.

UNSW Conjoint Professor David Ziegler and UNSW Conjoint Associate Professor Maria Tsoli led the study. They have been working for many years to find better treatments for DIPG.

"We recognize that no single drug treatment is able to eradicate the most aggressive of brain cancers on its own," says A/Prof. Tsoli.

She says this is what led the researchers to see if combining treatments could work better.

Prof. Ziegler says one of the biggest challenges with these tumors is that thousands of genes are switched on at the same time, driving the cancer's growth.

"It has proven extremely difficult to find a way to switch them all off," he says.

Two work better than one

The two treatments used in the study are both new-generation drugs called epigenetic therapies. These drugs affect how genes are turned on and off, without changing the DNA itself. They do this by interfering with a process called transcription, which is how cells read genes and make proteins.

Co- first author, UNSW Conjoint Lecturer Dr. Holly Holliday, says DMG cells grow out of control because of changes that disrupt normal gene activity.

"In this study, we discovered a promising combination of drugs that successfully shuts down the transcription process, effectively switching off thousands of genes at once," Dr. Holliday says.

The study focused on two important proteins involved in transcription: FACT and BET. These proteins are found at high levels in cancer cells. Drugs that block these proteins already exist, but when used on their own they only slow the cancer slightly.

When the researchers used both drugs together, they found the cancer cells died in laboratory experiments. The same treatment was then tested in mice, where it slowed tumor growth and helped the mice live longer.

The researchers also found the treatment activated signals linked to the immune system. This means the cancer cells may become easier for the body's immune system to recognize and target.

Because of this, the researchers think that adding an immune-based treatment, such as CAR T-cell therapy, could work even better in the future.

The road ahead

The study provides proof of principle—it shows the idea works in early testing but needs further studies before it can be applied.

Co- first author, UNSW Conjoint Lecturer Dr. Aaminah Khan, says both types of drugs are already being developed for use in patients, as they are now in clinical trials.

"The FACT inhibitor CBL0137 has already been tested in children and shown to be safe," Dr. Khan says.

"The next step is to identify the best BET inhibitor to use in combination with CBL0137. Once it is also shown to be safe in children, the team then plans to test the combination in a clinical trial for children with DMG."

While much more research is needed, the team hopes this work will one day lead to better treatment options for children with brain tumors.

Publication details

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