Researchers discover new way to treat deadly childhood brain cancer

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(Medical Xpress)—Researchers from the University of Toronto's Department of Laboratory Medicine and Pathobiology (LMP) have discovered a new way to effectively target a previously difficult-to-treat form of childhood brain cancer called ependymoma.

Professor Michael Taylor (MD,PhD, FRCSC), along with lead author Stephen Mack and co-authors including LMP Prof. Peter Dirks (MD, PhD), published the groundbreaking findings in *Nature* on February 19, 2014. Their research showed that epigenetics, the packaging of DNA, is the main cause of this cancer and can be targeted by FDA approved drugs.

DNA code is comparable to a three-billion letter long set of instructions on how a cell should operate. In this analogy, the majority of cancers are caused by words that are misspelled, words that are added or deleted or entire book chapters that are added or deleted.

"When we look at ependymoma, we don't find any misspelled words, deleted or duplicated words or book chapters that are missing. Instead, what we find is that the entire novel is written in the wrong font and the DNA is packaged improperly," said Prof. Taylor. "While epigenetics has been known to play a role in cancer, this is the first time that epigenetics is the prime driver of cancer."

Receiving a diagnosis of ependymoma, the third most common type of childhood brain cancer, can be devastating. While babies and toddlers

are treated with surgery and radiation therapy, chemotherapy has been ineffective and the cancer often recurs.

Impressively, there are already FDA approved drugs that can alter the cancer's epigenetics. "Usually when you make a discovery you have to make a new drug, but there are already drugs that change the font. In this case, there are drugs that are the Microsoft Word equivalent of select all and switch to Times New Roman," said Prof. Taylor.

The team's success relied heavily on their ability to grow and study cells from these rare tumours. Once a patient's tumour is removed from the operating room, its cells are taken to Prof. Peter Dirks's lab to be grown in specialized conditions. The process is so efficient that by the time a child has recovered from surgery, the team has tested the cells and will know whether a specific drug will work.

"I think that's where this project has been so exciting. Dr. Taylor and Steve Mack made a foundational discovery in this tumour, and we were able to bring our expertise together in growing these live cells to see whether targeting this packaging really had any promise," said Prof. Dirks.

The team is optimistic that this will provide hope for children suffering from ependymoma. Prof. Taylor explains, "We hope it will be an effective treatment. One drug doesn't usually result in a cure, but there are multiple drugs that attack the same mechanism. Even if we can't cure it, we could use the drug to keep it to a treatable disease like diabetes."

If the group receives funding, it could take only three to five years to complete a clinical trial.

More information: Paper: Epigenomic alterations define lethal CIMP-positive ependymomas of infancy, DOI: 10.1038/nature13108

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