

## Researchers from the Duke University say they have a breakthrough in treating cancer

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The latest research confirms results Duke's scientists have previously reported in experimental trials with brain cancer patients: Some patients have experienced dramatic shrinkage of brain tumors and remission of their cancer. Brain tumors are among the most aggressive forms of cancer with low survival rates; historically patients have been treated with surgery, radiation and chemotherapy.

If the results, published in the journal Science Translational Medicine, are further confirmed by additional tests and studies, they could ultimately lead to the development of therapies that could turn many forms of cancer into a manageable condition, like diabetes or HIV, said Matthias Gromeier, the lead researcher on the project and a neurosurgery professor at Duke Cancer Institute's Preston Robert Tisch Brain Tumor Center.

"We are starting to show that our drug can prolong survival rates in brain tumor patients," Gromeier said. "That's the hope – controlling the tumor."

Duke is among a number of leading research institutions nationwide making progress in developing cancer treatments that are expected to replace current treatment methods, which have toxic side effects and uncertain results. Broadly classified as immunotherapies, the new wave of treatments create customized medications using the patient's own cells, or train the patient's immune system to detect cancer, which is difficult to recognize by disease-hunting defender cells because cancer is an extension of the patient's body, not a foreign invader.

In February, UNC-Chapel Hill cancer researchers reported findings in Science Translational Medicine that showed engineered human stem cells can be deployed to identify cancer cells and travel toward them. The engineered stem cells could be used as delivery mechanisms that carry a deadly payload of medications targeting the cancer cells without harming healthy human tissue.

UNC brain cancer researchers are now moving ahead with the first attempt to attack cancer using engineered stem cells taken from patients' skin cells. The scientists hope to start enrolling patients for a clinical trial in the next 12 months.

Duke's researchers are deploying a polio virus that's genetically modified not to harm patients but naturally infects cancerous growths. The virus identifies the tumors for the patient's immune system, and activates the immune system to launch an attack.

The modified polio virus developed by Gromeier in 1994, received breakthrough therapy designation from the U.S. Food and Drug Administration last year. The designation, conferred upon experimental medications that show greater promise than current treatments for life-threatening conditions, allows the FDA to expedite the development and review of the drug.

But Duke's therapy is not without risks, Gromeier noted. When administered in excessive doses a polio virus injection can cause serious inflammation or other potential side effects, such as the patient's immune system going into overdrive. Some patients who were treated with the polio virus didn't fare well; they experienced severe swelling and their tumors spread unchecked.

Simon Khagi, director of the brain tumor program at UNC's Lineberger Comprehensive Cancer Care Center, said patients with cancer in advanced stages are already weakened by aggressive treatment that takes a toll on their bodies.

"You don't truly know if the patient died from the tumor or the immune response to the virus," Khagi said of the polio virus studies. "The problem with the immune system is, when you turn it on, it's really hard to turn it off."

Until they conducted their most recent study, which took more than three years to complete, Duke researchers intuited the cellular mechanism of their experimental treatment but lacked direct proof, because they could not take pathology brain samples from living patients. In this study, the researchers tested their hypothesis on mice infected with melanoma and on human breast cancer tissue in laboratories.

They say their approach can maintain a body's immune alertness that produces an effect similar to a vaccination, inhibiting the tumor from growing back after the immune system's initial attack.

"The virus can start an immune response, and we hope the immune system can eradicate the cancer," Gromeier said.

The Duke team's best-known patient is Stephanie Lipscomb, who was featured in a "60 Minutes" segment on the polio virus treatment. Lipscomb was diagnosed in 2010 at age 20 with a brain tumor the size of a tennis ball bulging behind her right eye. The tumor was surgically removed, but then later started growing back, and she agreed to be the first person injected with the polio virus formula. Subsequent tests showed her tumor shrank from the size of a lime to no larger than a green pea, allowing her to finish school and get a degree in nursing. She is healthy and doing well, Gromeier said.

Gromeier and other Duke scientists own the intellectual property related to their research project. The intellectual property has been licensed to Research Triangle Park-based Istari Oncology, of which Gromeier and Duke Pathology professor Darell Bigner are co-founders and shareholders.

Gromeier said Duke researchers are still several years away from applying for FDA approval to sell their treatment to hospitals and patients. Their first clinical trial, began in 2012, was designed to show the treatment is safe, and was tested on about 60 patients to determine a safe dose, but it did not prevent all the enrolled patients from succumbing to brain cancer.

Gromeier and his team are now enrolling patients in another clinical trial and planning a clinical trial for children with brain tumors. They eventually plan to test the therapy at other hospitals, one of the steps required to demonstrate the treatment is successful under a variety of circumstances.