# Brain cancer treatment shows promise in early trial

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(HealthDay)—An experimental viral treatment may extend the lives of

patients with a hard-to-treat brain cancer, researchers say.

For the phase 1 study, patients with recurrent glioblastoma, the most common and aggressive brain tumor, were injected with an engineered virus.

Survival was 13.6 months among 43 patients treated with the viral therapy, compared with 7.1 months for patients who did not receive the new therapy, according to the study.

"For the first time, this clinical data shows that this treatment, used in combination with an antifungal drug, kills cancer cells and appears to activate the immune system against them while sparing healthy cells," said study co-leader Dr. Timothy Cloughesy. He is director of the neuro-oncology program at the University of California, Los Angeles.

"This approach also has potential in additional types of the disease, such as metastatic colorectal and breast cancers," Cloughesy said in a university news release.

Cloughesy is also a consultant for Tocagen, the biopharmaceutical company that developed the therapy and funded most of the study.

Some patients who received the experimental treatment lived more than two years with few side effects, the researchers reported.

"Brain cancer is a deadly disease, and when it returns there are extremely few treatment options, and survival is usually measured in months," said study co-lead author Dr. Michael Vogelbaum, associate director of the brain tumor neuro-oncology center at the Cleveland Clinic.

Here's how the treatment works: Injectable Toca 511 infects actively dividing cancer cells and delivers a gene for an enzyme called cytosine

deaminase to the cancer cells. Inside the tumor, Toca 511 programs the cancer cells to make cytosine deaminase to set them up for the second step of the treatment.

In that next phase, the patient takes the antifungal drug Toca FC. The genetic changes triggered by Toca 511 cause the cancer cells to convert Toca FC into the anticancer drug 5-fluorouracil (5-FU).

This leads to the targeted death of infected cancer cells and cells that help tumors hide from the immune system, while leaving healthy cells unharmed, the researchers explained.

These are the first published clinical trial results of this new type of modified virus known as a retroviral replicating vector (RRV), according to the news release.

The purpose of a phase 1 study is to assess safety and tolerability. Three phases are usually required for a medication to receive U.S. Food and Drug Administration approval.

"The collective results from this virus study, include encouraging survival and excellent safety data, support the ongoing randomized phase 2/3 trial called Toca 5, and offer hope for a new treatment option for patients with brain cancer," Vogelbaum said.

The study results were published June 1 in the journal *Science Translational Medicine*.

**More information:** "Phase 1 trial of vocimagene amiretrorepvec and 5-fluorocytosine for recurrent high-grade glioma," *Science Translational Medicine*, stm.sciencemag.org/lookup/doi/ … scitranslmed.aad9784

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