

Fact Sheet



GIVING HELP, GIVING HOPE

Gene Therapy: A New Experimental Treatment for Brain Tumors

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How Does Gene Therapy Work?

Currently, for patients with malignant brain tumors who have a recurrence after surgery, radiation therapy, or chemotherapy, gene therapy may provide an experimental option for treatment.

Using gene therapy, researchers inject a substance into the brain tumor that changes the genetic makeup of the tumor cells. One study currently under investigation uses a substance which is manufactured in the laboratory and is called the TK gene. The TK gene is related to the herpes simplex virus, the same virus that causes cold sores, and it can be killed by an anti-viral drug called Cytovene® (ganciclovir sodium).

The problem is, simply injecting the TK gene into the substance of the tumor is not enough. It is necessary for this gene to enter the actual tumor cells so that the cells become infected and then can be treated with the anti-viral drug. To accomplish this, researchers have devised a method to place the TK genes into tumor cells using a vehicle called a vector.

The vector, manufactured from a different virus called a retrovirus, has been made harmless so that it cannot cause disease. But because it is a virus, it still has the power to infect cells. The vector and the TK gene are manufactured in a laboratory. When the vector/gene combination is injected into the tumor, the viral vehicle inserts itself into the cell carrying with it the TK gene as a hidden passenger.

Once inside the cell, the TK gene becomes part of the tumor cells' genetic material (chromosomes). When the cell replicates (reproduces), the TK genes are also replicated. In this way, the cells are tricked into manufacturing more of

the TK gene. Now that the tumor cells are producing TK genes, they are susceptible to Cytovene. (Recall that TK genes, related to the herpes simplex virus, are susceptible to the antiviral drug Cytovene.) Thus, when Cytovene® is later administered into the tumor, the drug kills the tumor cells that have been busy manufacturing TK genes.

The hope is that this therapy will not injure normal brain cells because the vector/gene combination is attracted to rapidly growing cells. Tumor cells grow rapidly and become targets for the vector/gene combination. Since normal cells do not grow rapidly, they may remain safe.

Who Can Undergo Gene Therapy?

Current clinical trials include patients with recurrent malignant gliomas or in some cases, patients with newly diagnosed malignant gliomas. Your physician or nurse can tell you whether you would be eligible for gene therapy clinical trial based on your individual situation.

What Are the Potential Side Effects?

In order to accurately inject the vector/gene cells, you will have to have stereotactic brain surgery. This procedure does carry risks as would any brain surgery. The risks of stereotactic brain surgery include hemorrhage (bleeding) and deterioration of neurological functions. The neurological problems could involve weakness in the arms or legs; loss of sensation over parts of the body; and partial or complete loss of functions related to communication, such as speech, comprehension, intellectual capacity, and memory. On the whole, however, the side effects are not very common.

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Three percent of patients who undergo stereotactic brain surgery experience the side effects of hemorrhage, infection, and neurological deficits.

Generally injecting vector/gene cells and administering Cytovene® do not cause the side effects of nausea and vomiting commonly associated with other therapies. But there have been some side effects, such as inflammation of the lining of the brain and infection, which have been associated with gene therapy. Your doctor will discuss to what degree these risks apply to you based on your condition, specific findings, and the planned surgical procedure.

How Do I Find a Gene Therapy Clinical Trial?

For a complete list of gene therapy clinical trials, please contact the National Brain Tumor Foundation at 1.800.934.CURE (2873).

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The National Brain Tumor Foundation (NBTF) was founded in 1981 as a non-profit organization by people whose lives were affected by brain tumor disease. NBTF provides support services for patients and their families and raises funds for research to treat and cure brain tumors. For more information call 800.934.CURE.

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