On the Research Front: How Gene Therapy and May Help Fight Cancer

What are genes?

Genes are the biological units of heredity. Genes are located on chromosomes inside cells and are made of deoxyribonucleic acid (DNA), which is a type of biological molecule. Genes carry the instructions that allow cells to produce specific proteins, such as enzymes. Each protein has a specific function. For example, some proteins help cells in the body to keep their shape, while others allow them to grow and divide normally. Flaws in genes can result in the loss of growth control of cells, resulting in cancer.

What is gene therapy?

Gene therapy is an experimental treatment that involves introducing genetic material (DNA) into a person's cells to fight disease. We are studying several ways to treat cancer using gene therapy. Some approaches target cancer cells, to destroy them or prevent their growth. In other approaches we target healthy cells to enhance their ability to fight cancer.

Why do we need a vector for gene therapy?

In general, a gene cannot be directly inserted into a person's cells. It must be delivered to the cells using a carrier, or "vector." The most commonly used vectors in gene therapy are viruses such as a common cold virus. Viruses have a unique ability to recognize certain cells and insert genetic material into them. We can alter the viruses used in gene therapy to make them safe for humans and to increase their ability to deliver specific genes to a patient's cells. To treat cancer effectively with gene therapy, we are modifying vectors that can be injected into the patient and specifically focus on the target cells located throughout the body.

How do gene therapy trials receive approval?

A proposed gene therapy trial, or protocol, must be approved by a review board at the scientist's institution. Gene therapy protocols must also be approved by the U.S. Food and Drug Administration (FDA), and by the National Institutes of Health (NIH) Recombinant DNA Advisory Committee. Any studies involving humans must be reviewed with great care

Will gene therapy be eventually used to treat cancer?

Although gene therapy is relatively new, it is potentially a very powerful technique and could have profound implications with the potential to save many individuals affected with cancer. There is little doubt that the techniques used in gene therapy will continue to improve and that gene therapy will eventually become an important way to prevent and treat cancer. Conducting clinical trials in patients to evaluate the safety and efficacy of gene therapy vectors is essential before this technology can be widely used to treat cancer.