What is Gene Therapy?

What is a gene and how does it work?

In the center of every cell in your body is an area called the nucleus. The nucleus contains your DNA (deoxyribose nucleic acid), which is the genetic code you inherited from each of your parents. Genes are made up of anywhere from a few hundred to a few million pieces of DNA. You have 46 chromosomes, which are each made up of thousands of genes. Each person has between 20,000 and 25,000 genes in their body. You have 2 copies of every gene, 1 inherited from your mother and 1 from your father.

Genes contain instructions for the cell on how to make proteins. Proteins are necessary to perform most life functions. Genes tell cells how to work, control our growth and development, and determine what we look like and how our bodies work. They also play a role in the repair of damaged cells and tissues.

What is gene therapy?

So, now that we know what genes are, how can they be used to help fight cancer? If a gene becomes damaged, this damage is called a mutation. This can lead to a gene not functioning properly and a cell growing uncontrollably. This can eventually lead to cancer formation. Keep in mind that developing a cancer is not quite as simple as this, but requires a complex series of multiple mutations. These mutations may be caused by things like smoking, the environment, or they may be inherited. It makes sense that if we could repair these mutations, we could potentially stop a cancer from starting. The question is, how do we do this?

Researchers are testing several ways of applying gene therapy to the treatment of cancer:

- Replace missing or non-functioning genes. For example, p53 is a gene called a "tumor suppressor gene." Its job is just that: to suppress tumors from forming. Cells that are missing this gene, or have a non-functioning copy due to a mutation, may be "fixed" by adding functioning copies of p53 to the cell.
- Oncogenes are mutated genes that are capable of causing either development of a new cancer, or the spread of an existing cancer (metastasis). By stopping the function of these genes, the cancer and/or its spread may be stopped.
• Use the body's own immune system by inserting genes into cancer cells that then trigger the body to attack the cancer cells as foreign invaders.
• Insert genes into cancer cells to make them more susceptible to or prevent resistance to chemotherapy, radiation therapy, or hormone therapies.
• Create "suicide genes" that can enter cancer cells and cause them to self-destruct.
• Cancers require a blood supply to grow and survive, and they form their own blood vessels to accomplish this. Genes can be used to prevent these blood vessels from forming, thus starving the tumor to death (also called anti-angiogenesis).
• Use genes to protect healthy cells from the side effects of therapy, allowing higher doses of chemotherapy and radiation to be given.

How is gene therapy given?
Gene delivery is one of the biggest challenges to successful gene therapy. You can imagine it would be hard to actually inject these genes into the tiny cells, so a carrier, or a "vector," is used to accomplish this. Typically, viruses are used as the vectors. The virus vector must be genetically altered to carry human DNA. These viruses are like those that cause the common cold, only they are "deactivated" so that they will not cause the patient to actually get the cold.

In some cases, some cells are taken from the patient and the virus is exposed to the cells in the laboratory. The virus with the desired gene attached finds its way into the cells. These cells are allowed to grow in the laboratory, and are then given back to the patient by intravenous (IV) infusion or are injected into a body cavity (i.e. the lung) or a tumor. In other cases, the vector with the attached gene is directly inserted into the patient by intravenous infusion or is injected into a body cavity or a tumor. Once the gene has reached the cell, it must go to the cell's nucleus and become integrated within the human genetic material. Then it needs to be "turned on," to produce the protein product encoded by the gene. For gene delivery to be successful, the protein that is produced must function properly. As you can see, that's a number of steps that need to go right for the therapy to work.

What are the side effects of gene therapy?
Given that gene therapy is so new, we do not know all the side effects it may have, particularly long-term side effects that may occur years after receiving this therapy. After initially receiving a type of gene therapy, the patient's immune system may react to the foreign vector, causing fever, severe chills (called rigors), drop in blood pressure, nausea, vomiting, and headache. These symptoms typically resolve within 24-48 hours of the infusion. Other side effects are dependent upon the type of vector used and how it is given. For example, if the gene is given into a patient's lung, the side effects may affect the lung.

Some side effects are theoretical, meaning that it is possible that they could happen, yet they have not actually happened in clinical trials as of yet. There is a fear that the genes could enter healthy cells, causing damage to them, which could then lead to another disease or another cancer. If genes enter reproductive cells, they could potentially cause damage to sperm or eggs. It is feared that this damage could then be passed on to future generations. At this time, researchers are particularly careful to monitor for these unwanted complications, and perform tests in animal studies before any particular therapy is given to humans.

Resource for more information


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