

Gene Therapy - Weighing the Risks and Benefits

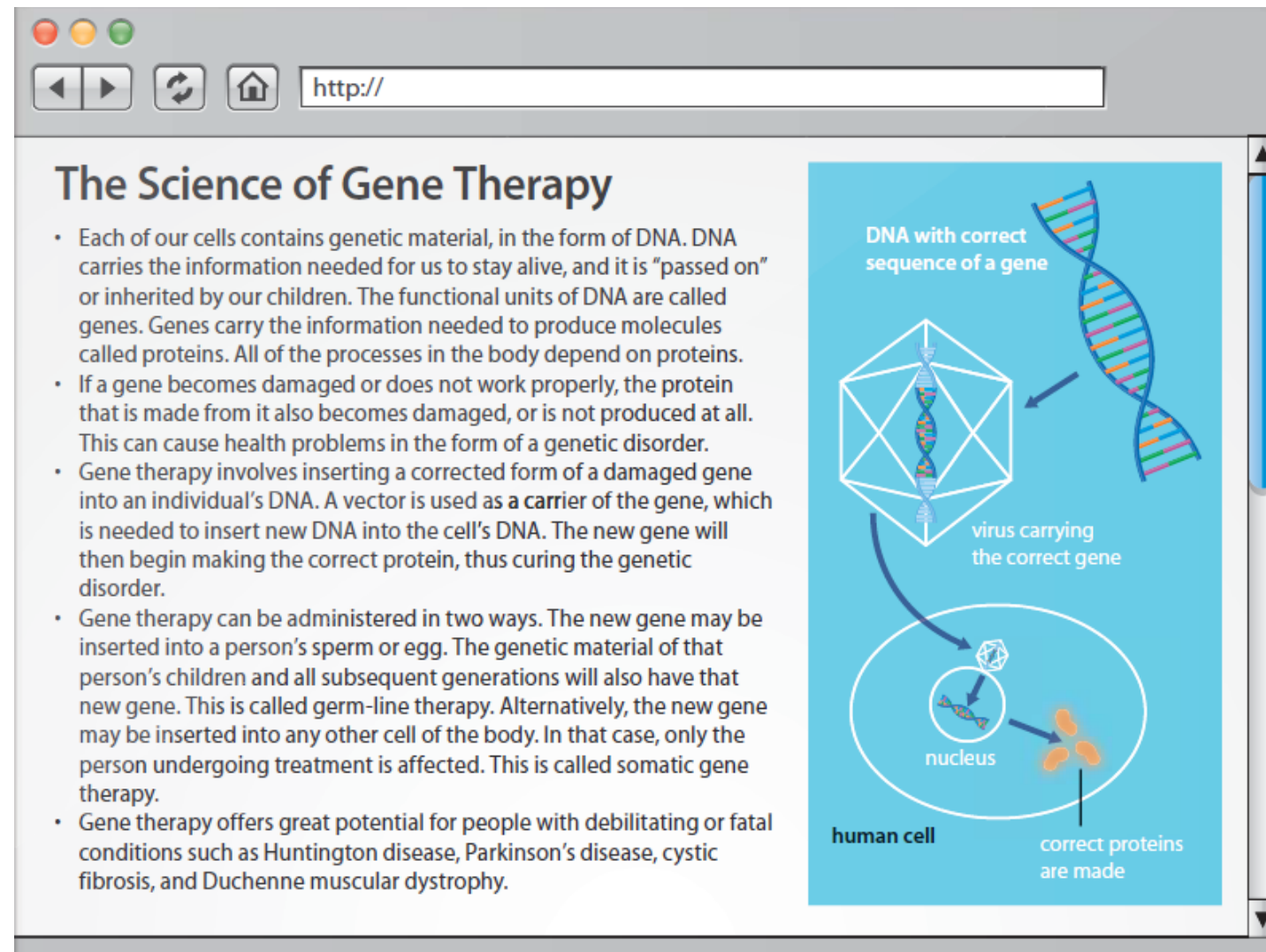
Scenario

To meet the 40-hour community involvement requirement for your graduation diploma, you are volunteering at a local hospital. One of your first assignments is in a clinic for people who have genetic disorders. The patients and their families come to the clinic to meet with their doctors and evaluate their treatment. A key role of this clinic is to facilitate meetings between patients and physicians who are investigating alternative treatments for genetic disorders.

One treatment that physicians are researching is gene therapy. Many patients are nervous when they come to the clinic. They have questions and concerns, especially about new experimental techniques. The hospital has developed a website (below) that describes the science of gene therapy. However, some people are concerned about the clinic's research into gene therapy and have developed a handout to publicize their concerns.

Your role is to read the information provided by these groups, as well as perform further research on the topic. From this, you will analyze the risks and benefits and develop an information brochure that could be used in the clinic.

READ THE TWO FOLLOWING IMAGES BEFORE STARTING



The Science of Gene Therapy

- Each of our cells contains genetic material, in the form of DNA. DNA carries the information needed for us to stay alive, and it is "passed on" or inherited by our children. The functional units of DNA are called genes. Genes carry the information needed to produce molecules called proteins. All of the processes in the body depend on proteins.
- If a gene becomes damaged or does not work properly, the protein that is made from it also becomes damaged, or is not produced at all. This can cause health problems in the form of a genetic disorder.
- Gene therapy involves inserting a corrected form of a damaged gene into an individual's DNA. A vector is used as a carrier of the gene, which is needed to insert new DNA into the cell's DNA. The new gene will then begin making the correct protein, thus curing the genetic disorder.
- Gene therapy can be administered in two ways. The new gene may be inserted into a person's sperm or egg. The genetic material of that person's children and all subsequent generations will also have that new gene. This is called germ-line therapy. Alternatively, the new gene may be inserted into any other cell of the body. In that case, only the person undergoing treatment is affected. This is called somatic gene therapy.
- Gene therapy offers great potential for people with debilitating or fatal conditions such as Huntington disease, Parkinson's disease, cystic fibrosis, and Duchenne muscular dystrophy.

The diagram illustrates the process of gene therapy. It shows a DNA double helix with a section labeled "DNA with correct sequence of a gene". A "virus carrying the correct gene" is shown injecting its genetic material into a "human cell". Inside the cell, the "nucleus" is shown with the inserted gene. The final result is that "correct proteins are made".

Gene Therapy: Genetic Engineering of People!

- Genetic engineering involves changing an organism's genetic material. Gene therapy is a form of genetic engineering that is applied to humans.

- A commonly used vector is a virus. There is no guarantee that the virus will find the correct point in the person's DNA. If the gene is inserted in the wrong place, it will cause further errors in the genetic material. This could result in even worse consequences and the individual becoming even more ill.

- The immune system of the person undergoing treatment may attack and destroy the virus. In some cases, patients may need to go through many gene therapy sessions. This may cause the immune system to develop more and stronger responses to the virus.

- Gene therapy opens the door for many disturbing applications of genetics. The potential to use gene therapy so that all subsequent generations are affected is a way of making "designer babies." The gap between wanting a healthy child, free of a genetic disorder, and wanting to design a child to be a musical or mathematical genius is not very wide.

- Some people feel that meddling with the fundamental structure that gives each person his or her uniqueness is equivalent to "playing God."

Dr. A Delgado
for the concerned members of our community

