Gene Therapy
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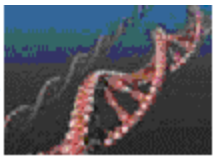
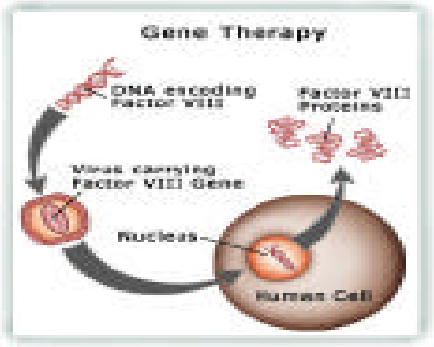


Over the past decade gene therapy has become an important medical topic. Genes are the physical and functional units of heredity. Found on DNA, they carry instructions for making proteins, which perform most of life's functions. When a gene is defective, the protein does not function properly or is not even created. If this protein is important in the body, a genetic disorder will result. About 1 in 10 people has or will develop a genetic disorder. However most people carry defective genes but remain unaware of them. This is because we carry almost two copies of each gene (one from the mother and one from the father). If one gene is defective, in most cases, the other normal copy will counteract it and take over the responsibilities.

Today there are about 2,800 conditions known to be caused by defective genes. Gene therapy is the technique to replace the defective gene with a normal gene. The way this works is that a carrier molecule, called a vector, is used to transport the normal gene into a patient's target cell. The most common vector being used is a virus which has been altered to carry human DNA. Once this vector is in the patient's cell, the new genetic information is loaded. The normal gene would begin to generate protein and the cell returns to its normal state.

Currently gene therapy is still in its trial state and no product is available for purchase. From the first clinical trail in 1990, gene therapy has had many unsuccessful attempts. One such case was Jesse Gelsinger, who died four days after treatment. His death was due to organ failures. From the beginning of these trails the FDA has had a strong impact. These trails have resulted in some success and improvements. The most recent improvement was made by laser scientists. They developed a new technique of inserting genetic material into a patient's cell. This technique involves using a miniature violet laser to make a small incision in the patient's cell membrane. Once the membrane is open a new gene can be inserted and the cell's internal mechanism allows it to self-heal. If the insertion is successful the new gene would generate protein.



Gene therapy research has many difficult problems ahead. One problem is the long run effects of the new DNA inserted into cells. Trials have yet to result in the life and stability of this DNA. Another problem therapy faces is the immune system. Since the job of the immune system is to rid the body of foreign objects, it will attempt to rid the body of the new genes. Also, most trials have been targeting a disorder caused by a single gene but scientists face problems with treatments for multiple gene disorders.

References:

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