Gene Therapy Using Viral Vectors

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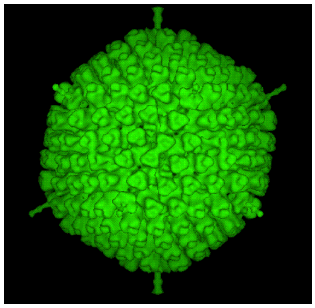
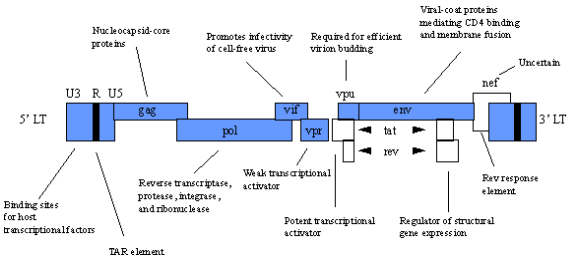
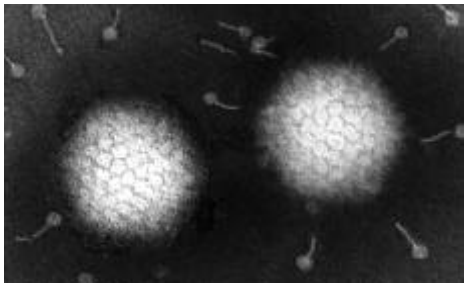
Gene therapy is a method of treating diseases by introducing normal genes into patients to overcome the effects of defective genes. It began in 1990 using *ex vivo* techniques. *Ex vivo* or the indirect method of gene therapy starts with removal of the patient's cells. They are then cultivated in a laboratory and incubated with vectors, which are gene delivery vehicles that encapsulate therapeutic genes for delivery to cells, to modify their genes. The cells are then transplanted back to the patient where they multiply and carry out their function. The other method of gene therapy is *in vivo* or the direct method. By this, the normal genes are inserted into virus as the vector. When the virus is inoculated to the patient, it searches out the specific cell and transplants its viral DNA (now the normal patient DNA) into that cell.



Retroviruses were the first viruses used as vectors and are today the most common. They have strands of RNA ranging for 3.5 to 10 kilobases long. The most common retrovirus used, and has the simplest genome, is the Moloney murine leukemia virus (MMLV). This virus only infects rapidly dividing cells, which makes it useful in the fight against cancer.



The human immunodeficiency virus (HIV) has one of the more highly complex genomes. It is used in the treatment of aquired immunodeficiency syndrome (AIDS). It can selectively target HIV virus containing cells. A HIV vector is administered to the patient. It then searches out HIV containing cells, infects it, and subsequently the cell dies, along with it the HIV virus. The adeno-virus is the cause of the common cold, respiratory infection, and conjunctivitis to name a few. Their highly complex genome can range from 36 to 38 kilobases long. It was first used in the treatment of cystic fibrosis (most common fatal genetic disease). A common cold virus is used to replace a defective gene that codes for a sodium and chloride transporter. The vector is inhaled into the lungs where it infects the cells. The cells, with the normal DNA, produce the transporters.



Gene therapy is on the cutting edge of medical science today. When the procedures for gene delivery are more dependable the possibilities will be endless in the fight against disease.