A Closer Look at Gene Therapy for Cystic Fibrosis

In people with cystic fibrosis, a protein called CFTR is absent from cells in the lungs. Without this protein, mucus builds up in the lungs and causes many of the symptoms of the disease. Gene therapy experiments were developed to attempt to treat cystic fibrosis. The process, which is illustrated in the figure below, involved genetically engineering a cold virus so that it could produce the CFTR protein. The virus was then delivered to the patient’s lungs through a tube inserted through the mouth or nose.

A Modified viruses carry a gene for the CFTR protein that is missing in people with cystic fibrosis.

B After the virus attaches to a lung cell, the cell membrane begins to surround the virus.

C Cell enzymes break down the outer coat of the virus, and the viral DNA moves into the cell nucleus.

D In the nucleus, a strand of messenger RNA is produced by copying the genetic information in the viral DNA. The messenger RNA moves into the cytoplasm.

E The cell’s ribosomes use the messenger RNA to produce the CFTR protein.

Answer the following questions on a separate sheet of paper.

1. What role does CFTR play in the body?
   Prevents mucus from building up in lungs.

2. Why is it necessary for the viral DNA to enter the cell’s nucleus before it can do its job?
   Because by phase of protein synthesis takes place in the nucleus (copying DNA to mRNA).

3. Where in the treated cells is CFTR actually produced?
   In the ribosomes in the cytoplasm.

4. The cold viruses used in gene therapy for cystic fibrosis are genetically engineered so they cannot reproduce, and thus cannot cause a viral infection in the patient. Because of this, the therapy does not lead to a permanent cure for cystic fibrosis. Explain why this is the case.

   When the "fixed" lung cells die, no more CFTR is produced. New cells that replace them will still have defective DNA code and the treatment will have to be repeated.