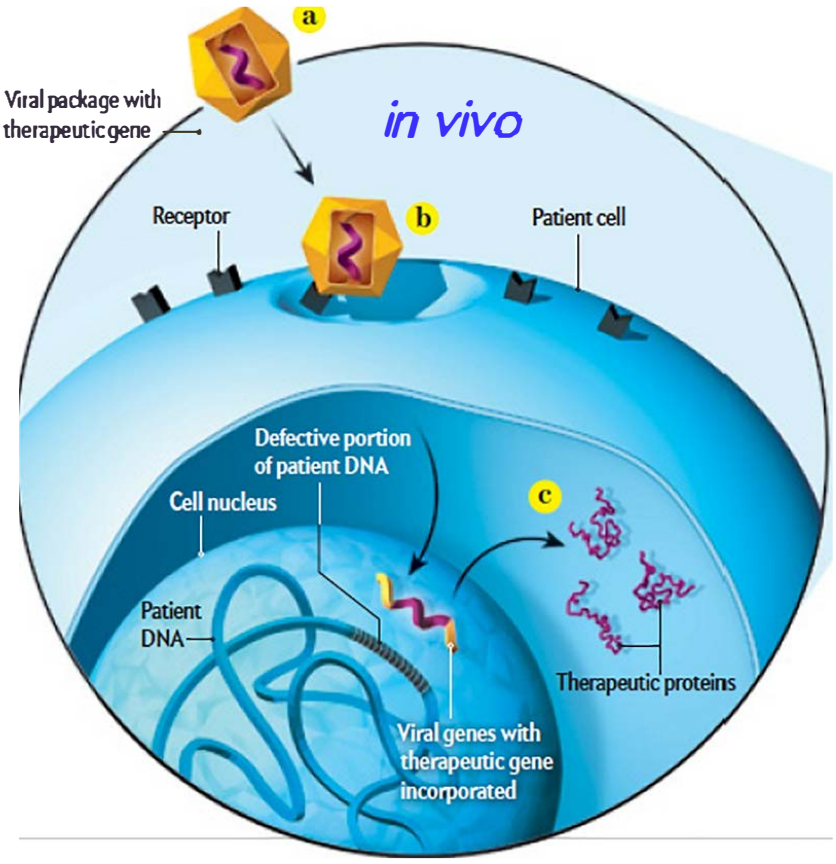
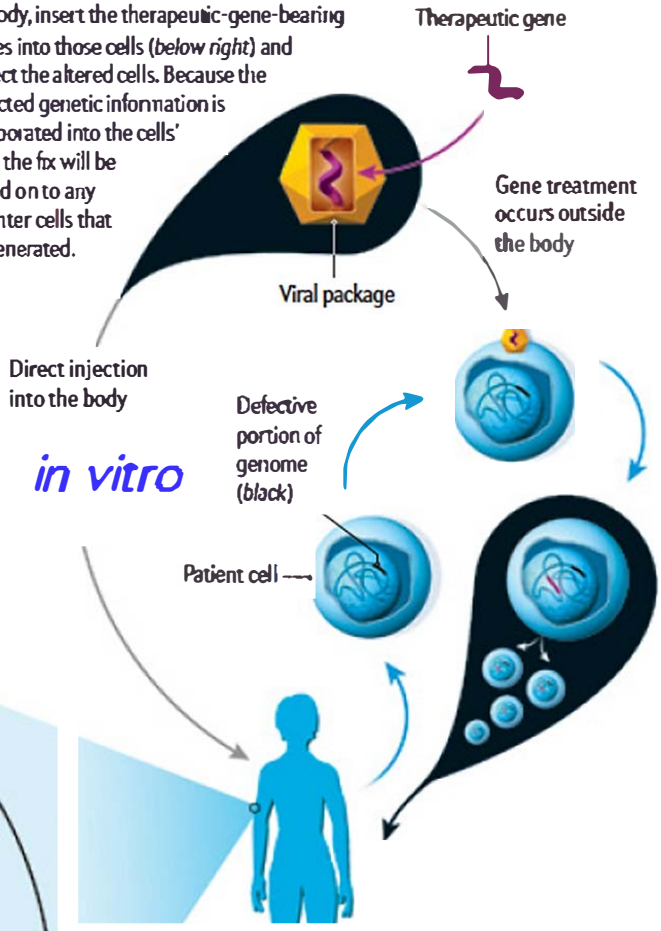
Viral Vectors

Two delivery choices in vivo & in vitro

Gene therapy attempts to undo the damage caused by broken or defective genes. The most common approach (*below*) packages a copy of a working gene into a virus **a** that has been stripped of most of its original content. This hybrid virus with its therapeutic payload is then injected into the body, where it attaches to receptors **b** on targeted cells. Once inside a cell, the corrected copy of the gene instructs the cell to start manufacturing the protein **c** that it had previously been unable to pro-duce. Unwanted side effects may occur if genes are acci-dentally inserted into the recipient's genome in a way that causes cancer or if the patient's own immune sys-tem tries too vigorously to defend the body against what it determines to be a foreign invasion (*not shown*).



In addition to injecting viruses into patients directly, investigators may remove cells from the body, insert the therapeutic-gene-bearing viruses into those cells (*below right*) and reinject the altered cells. Because the corrected genetic information is incorporated into the cells' DNA, the fix will be passed on to any daughter cells that are generated.



Enhancing Safety

Researchers minimize the chances of cancer or a dangerous immune attack by carefully choosing the type of viruses they use, limiting their number or restricting the tissues that are treated.