

Bubbles Away!

Learning Objectives

- Basic understanding of gene therapy linked to a clinical example.
- Replacement genes and vectors.
- Outline of steps involved in therapy.

Answers

1. A replacement gene is a piece of DNA containing a healthy or "normal" copy of the gene that is altered/mutated in the patient. This piece of DNA has been cut and pasted into a virus. The virus has been engineered to remove the pathogenic components so that the virus now acts as a carrier or "vector" for transporting the replacement gene into human cells and will not produce a viral illness in these cells.

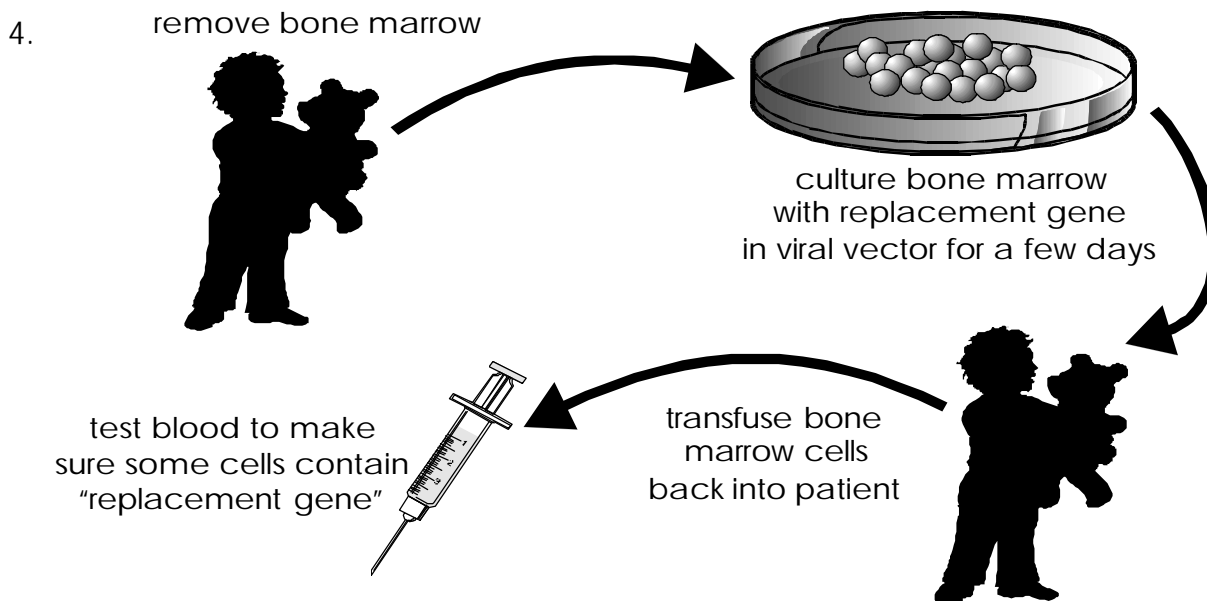
2. The advantages of using a viral delivery system are that:

- the virus is able to use receptors on the cell surface to gain entry into the cell in a very effective manner;
- viruses are able to target specific cells.

The disadvantages are that:

- viruses may cause inflammatory responses;
- there are concerns about their long term stability and safety.

3. Liposomes are like fat droplets which form a liposome/DNA complex when mixed with DNA and are absorbed through the cell membrane.



5. Somatic gene therapy only alters the cells of the particular tissue into which the gene has been inserted. Germline gene therapy alters all the cells of the body which can potentially alter genes in subsequent generations.