Vivet THERAPEUTICS

A GUIDE TO GENE THERAPY

This leaflet will provide you with information about **what gene therapy is, how it works** and **how it might help someone who has a genetic disease**

What are cells and genes?

Cells are the building blocks of all living things and come in many different forms, such as skin cells, muscle cells and nerve cells.[1] The human body is made up of trillions of cells, each with their own unique functions.[1]

Almost every cell in our body contains a molecule called DNA, which carries all the information we need to develop, grow, reproduce and live.[1,2]

Genes are specific segments within a DNA molecule. Genes provide the instructions needed for creating proteins.[1] Proteins are molecules that are necessary for our bodies to perform basic functions.[2] We can therefore think of DNA as a 'recipe book', and of each gene as a specific 'recipe' for a protein.

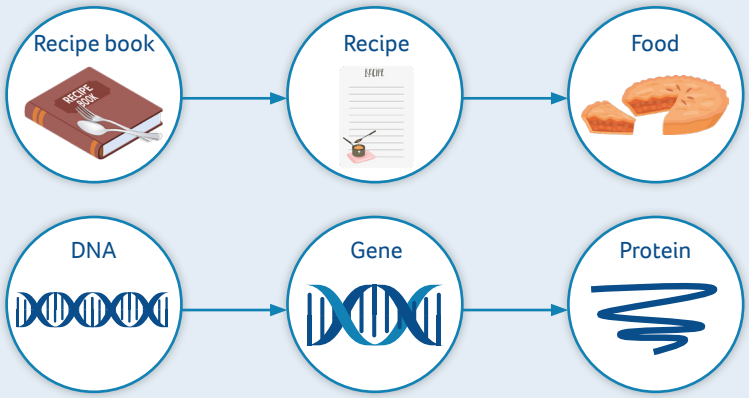
DNA is stored as a code of four chemical bases called A, T, C and G, arranged into a structure called a double helix.[1]

Genes are segments within a DNA molecule

We inherit two copies of every gene, one from each of our parents, and each gene has different versions.[1] For example, the genes for eye color can be the 'brown' or 'blue' versions.

This happens because the 'recipe' is slightly different between the two versions of the gene.[3]

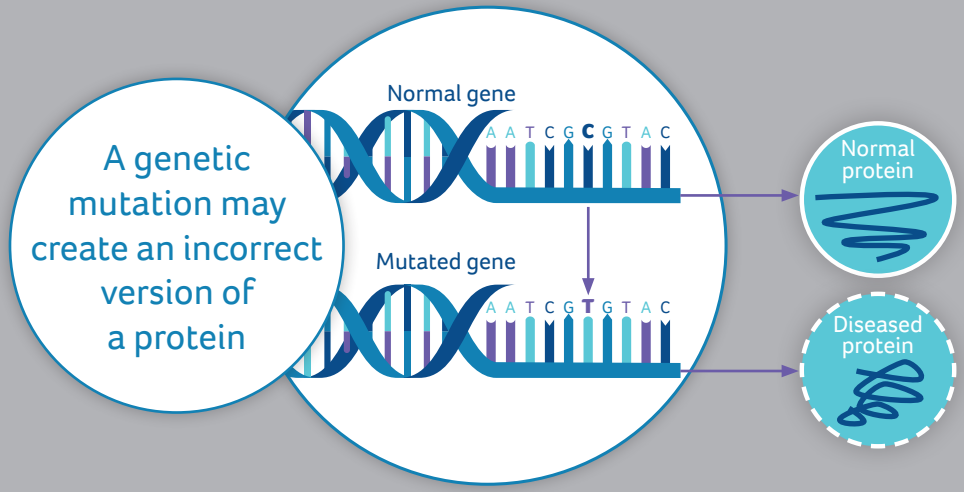
Genes can be thought of as 'recipes' for proteins



What is a genetic disease?

A genetic disease or disorder occurs when there are changes (called 'mutations') in a person's DNA that affect a gene.[3] Mutations can be inherited from one or both parents or may just happen by chance. Not all mutations are harmful. Some mutations cause harmless differences in our physical appearance, such as height or eye color. However, in genetic diseases, a mutation creates a protein that doesn't work correctly in our bodies.[3] This may lead the affected individual to experience a range of symptoms.

The order of the A, T, C and G letters in DNA is important, just like the letters in a word.[1] An error in this DNA code, even in one letter, is known as a mutation.[3] A genetic mutation may create an incorrect version of a protein, which can cause disease[3]



What is gene therapy?

Gene therapy is a medical technique by which a gene is modified to treat a given genetic disease.[4] This may involve adding the correct gene to cells or correcting the gene that is already there.

For a gene to enter a cell, it needs to be packaged into a delivery vehicle, otherwise known as a 'vector'.[4]

Viruses work well as vectors because they can easily enter our cells. Scientists can create special versions of viruses for this purpose. These 'special' viruses are harmless and won't cause disease when used in people.[4] To do this, scientists replace the DNA that is already present in the virus with the gene that will be used to treat the disease.

How is gene therapy given to patients?

With gene therapy, cells can be treated inside the body (*in vivo*) or outside the body (*ex vivo*).[5]

- If the cells can be treated inside the body, the vector may be, for example, infused into the blood. This is similar to receiving medicine through an intravenous drip.[5]
- Cells that need to be treated outside the body will be removed and treated with the vector, before being infused back into the blood.[5]

What are the potential *benefits* of gene therapy?

Once a gene therapy procedure is complete, the cells will contain a new healthy gene, allowing them to produce a protein that works correctly.[6] This may eliminate or reduce the symptoms of Wilson's disease and/or avoid the need for long-term medications or dietary changes.

Are gene therapies currently used as treatments?
Active research on gene therapy started about 50 years ago, with several gene therapies becoming available for patients in recent years.[6]

What are the potential *risks* of gene therapy?

As with any treatment, a gene therapy may not provide the expected benefit and/or may be associated with side effects.[4]

In addition, although gene therapies have recently demonstrated clinical benefits that last for several years in patients,[7,8] it is not known how long these effects will continue.

Please ask your physician for more information about gene therapy.

For further information about gene therapy:

The American Society of Gene & Cell Therapy (ASGCT) has created a series of online videos to explain the basics of gene therapy. You can find these at https://www.asgct.org/education/gene-therapy-101

References:

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