

UNDERSTANDING GENE REPLACEMENT THERAPY

Gene therapy is a scientific technique that uses a working gene to treat or prevent diseases.

Gene replacement therapy is a type of gene therapy that uses a “new gene” to replace a faulty or missing gene. This approach attempts to address the root cause of genetic diseases.

What is a genetic disease?

Genes tell the body how to make important proteins that it needs to function. Genes come in pairs—one from each parent. A genetic disease is caused by a faulty or missing gene or genes. A genetic disease can be passed down from one or both parents or can be a result of random errors in the body’s genes.

What is a monogenic disease?

A monogenic disease is a disease caused by a single faulty or missing gene or gene pair. This can cause a problem in the body’s ability to make a needed protein. Nearly every function of the body is made possible by proteins. So, even if a single protein is missing, in short supply, or made wrong, the impact on the body can be very harmful.

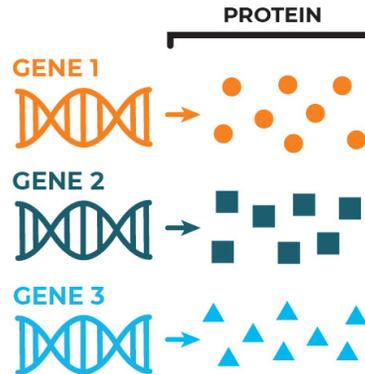
We believe monogenic diseases could be ideal candidates for a gene replacement therapy treatment because they are caused by one gene.

What is the goal of gene therapy?

Gene replacement therapy has the potential to help people with monogenic diseases.

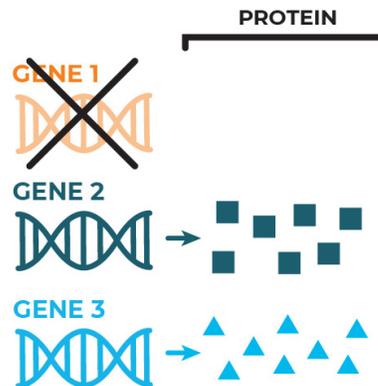
PERSON WITHOUT A MONOGENIC DISEASE

Usually a person's own genes each do their job to produce specific proteins.



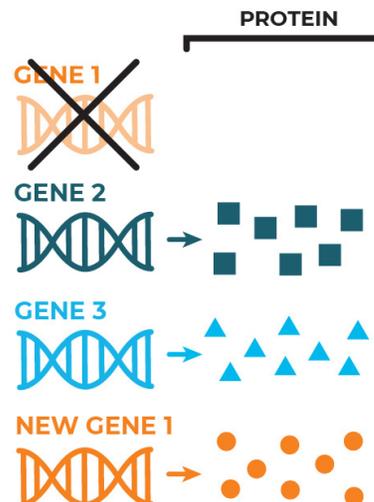
PERSON WITH A MONOGENIC DISEASE

In a person with a monogenic disease, there is one gene that is missing or does not work right. This results in either the protein being made incorrectly, made in short supply, or not made at all.

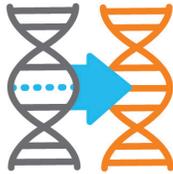


PERSON TREATED WITH GENE REPLACEMENT THERAPY

The goal of gene replacement therapy is to give the body a new, working copy of the missing or faulty gene. This new gene may or may not become part of a person's DNA and is able to give the body instructions for making a particular protein the body needs.



How does gene replacement therapy work?



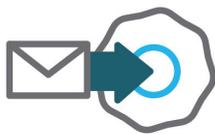
1. Gene replacement therapy starts with scientists creating a new, working copy of a missing or faulty gene.



2. Then the new gene is placed inside a vector. A vector acts like an envelope. It carries the gene to the right places throughout the body.



3. A vector can be created by making changes to a naturally occurring virus. A virus is selected as a vector because of its ability to enter the body. One such virus, called an adeno-associated virus, or AAV, is used because it is not known to cause sickness in people.



4. Next, the vector enters the body and carries the new gene to the control center of the cells, also known as the nucleus.



5. Once inside the nucleus, the new gene immediately goes to work to tell the body how to make the protein it needs. Finally, the rest of the vector is broken down by the body.

A historical look at gene therapy

Gene replacement therapy takes a different path to potential treatment because it addresses the root cause of the disease. Gene therapy, however, is not a new concept. Scientists have been exploring it as a way to treat genetic illness for decades.

A TIMELINE OF PROGRESS



Continued on next page.

A TIMELINE OF PROGRESS (CONTINUED)



2003

China approves the first gene therapy in the world for head and neck cancers.



2009

Scientists show that an AAV vector has the potential to cross the blood-brain barrier in the body. This is a major advancement toward the treatment of genetic diseases with gene replacement therapy.



2012

Over 1,800 gene therapy clinical trials have been completed, are ongoing, or have been approved worldwide at this point.



2017

A gene therapy is approved for a genetic disease that causes blindness.



2018

Many gene therapies for treating genetic diseases are being investigated in clinical studies.

As gene therapy continues to advance, the possibilities grow closer for people living with genetic diseases.

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