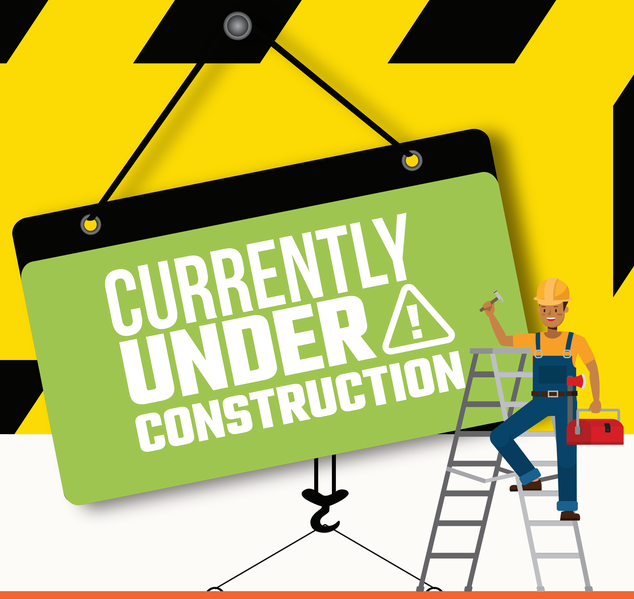
TREATMENT OPTIONS OF TOMORROW



Gene therapy is an umbrella term for what's known scientifically as either gene transfer or gene editing. With respect to hemophilia, most of the science being explored falls under the category of gene addition, where a gene is added to the body in order to replace the function of a gene that isn't working.

Did You Know?

- Only about 1% of DNA is made up of protein-coding genes, such as **FACTOR proteins**; the other 99% of DNA is made up of genes that serve other functions.
- As the host cell replicates the working **FACTOR** gene, the liver will continue to produce the non-working **FACTOR** gene.
- While exploring gene therapy, science is continuing to seek ways to improve factor replacement therapy, as well as looking at other ways to counterbalance the bleeding and clotting systems of the body.

What is your bottom line about gene therapy in 2019?

Gene therapy, in one sense, is a type of long-acting factor treatment. It's based on using a person's own mechanisms for creating clotting factors. Many view gene therapy, as it exists in 2019, as the beginning of a gene correcting/editing/delivery era.

Dr. Michael Tarantino, Founder & Medical Director, Bleeding & Clotting Disorders Institute

Gene therapy is a reality, it is just a matter of "when," but I don't think a blanket approval will happen here. Safety and efficacy questions remain. Variability and durability are unknowns. We don't know about the health of the liver [over time]. That is my biggest concern.

Dr. Michael Wang, HTC Clinical Director, University of Colorado

The first gene therapy [treatment option] for Hemophilia A will be filed with Food and Drug Administration (FDA) in 2019. This is a major milestone.

Dr. Glenn Pierce, World Federation of Hemophilia, Vice President, Medical

1. Our story begins with a **virus**! A virus is a small agent or particle that replicates only inside the living cells of an organism, is capable of binding to a cell, and can carry genetic material to a cell.
2. The virus is then stripped of its existing genetic material, leaving behind an empty shell, known as a **vector**.
3. The vector is implanted with a working **FACTOR** gene. The virus has now become what's known as a **viral vector**.

4. The viral vector is injected into the body with explicit instructions to go to the **liver**.
5. Once in the liver, the viral vector binds to a cell, which then becomes the **host cell** for the gene the viral vector was carrying.

6. The host cell will now start to produce **FACTOR proteins**.
7. The host cell will now **reproduce itself** with the working **FACTOR** gene, so that new cells will also produce **FACTOR** proteins.

www.TheScienceFair.org

BelieveLIMITED

EVENT HASHTAGS
#TheScienceFair #BelieveInScience

FOLLOW US
facebook.com/believescience @believescience