HEMHORIZON

WHERE COULD HEMOPHILIA TREATMENT GO NEXT?

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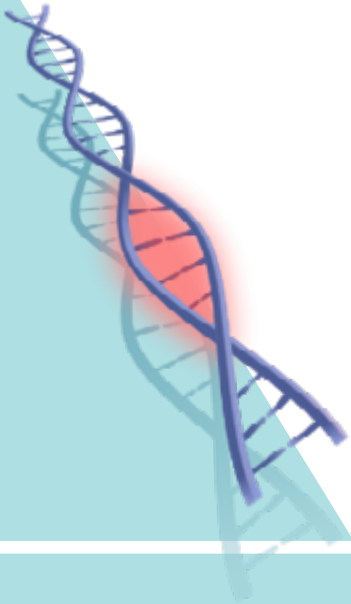
About Hemophilia

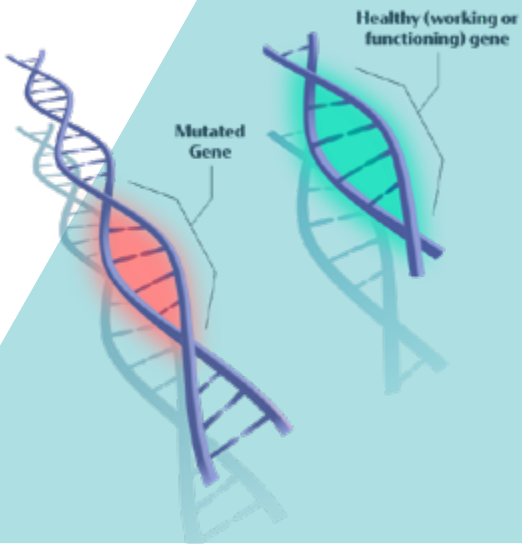
Hemophilia is caused by a mutation in a single gene. In hemophilia A, the F8 gene is mutated, so the body produces non-functional, limited, or no factor VIII protein. In hemophilia B, the F9 gene is mutated, and the body produces non-functional, limited, or no factor IX protein.

These proteins, known as clotting factors, work together to help blood clot properly and stop bleeding. If there is a low level of either clotting factor, the necessary steps needed to stop bleeding are impacted.



Scan for more about hemophilia, or visit HemHorizon.com/about-hemophilia.

Scan to continue reading about the potential of gene therapy, or visit HemHorizon.com/potential-gene-therapy.



Potential of Gene Therapy

There are different approaches to genetic medicine being studied across various therapeutic areas today. In hemophilia, most clinical trials have focused on gene transfer or addition, which is the transfer or addition of genetic material into the cell. This resource is focused on gene therapy (transfer or addition).

Gene therapy (transfer or addition) doesn't just aim to treat symptoms of a disease or disorder. Instead, gene therapy can potentially help treat certain diseases by introducing a healthy (working or functioning) gene that may improve the burden of hemophilia treatment.

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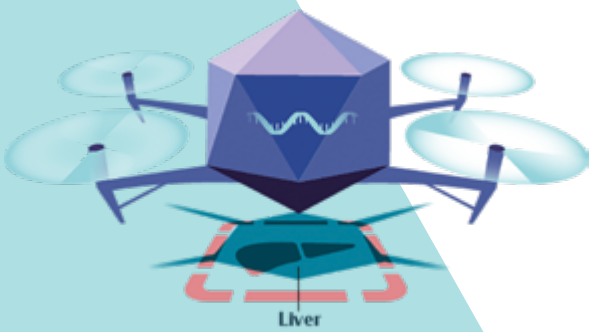
Science of Gene Therapy

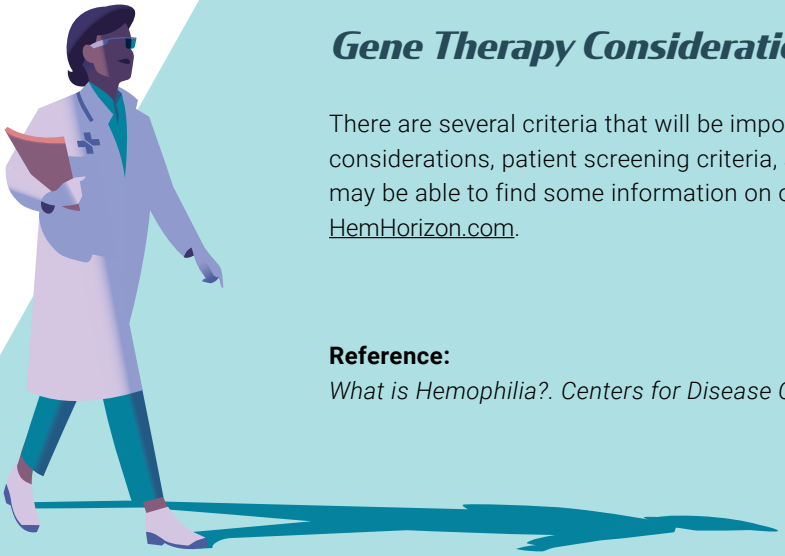
In gene therapy (transfer or addition), a carrier shell delivers a healthy (working or functioning) gene, which is introduced into the cells of a person to treat a specific medical condition. When carriers contain healthy genes, they are called vectors. Vectors are essentially vehicles designed to deliver healthy genes directly into target organs, such as the liver.



If you're interested in the science of gene therapy, scan here to continue reading, or visit HemHorizon.com/science-gene-therapy.

While gene therapy introduces a healthy gene to a person's body, it does not replace or edit the mutated gene or change a person's DNA. The healthy gene simply provides the body with the information that is needed to produce the missing or non-functional protein. In the case of hemophilia, the healthy gene provides the information needed for the body to produce clotting factor protein VIII or IX. Since a person's DNA is not changed after gene therapy, people treated with gene therapy may still pass on genetic mutations and conditions to their children.

Scan here to learn more about safety considerations, or visit HemHorizon.com/considerations.



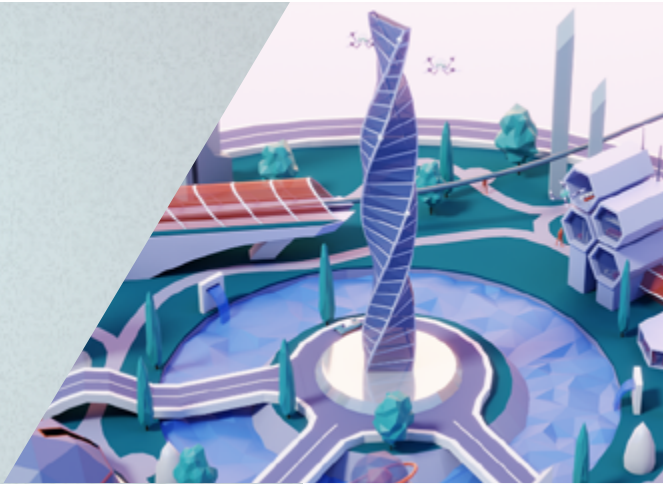
Gene Therapy Considerations

There are several criteria that will be important to consider, such as safety considerations, patient screening criteria, and gene therapy length of effect. You may be able to find some information on our website, HemHorizon.com.

Reference:
What is Hemophilia?. Centers for Disease Control and Prevention. Accessed 04-30-24.

Discover what may be on the horizon for hemophilia treatment.

To learn more visit HemHorizon.com.



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