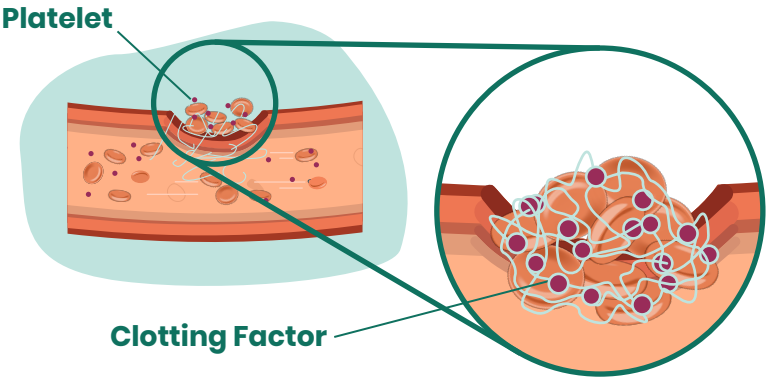
Hemophilia and Gene Therapy

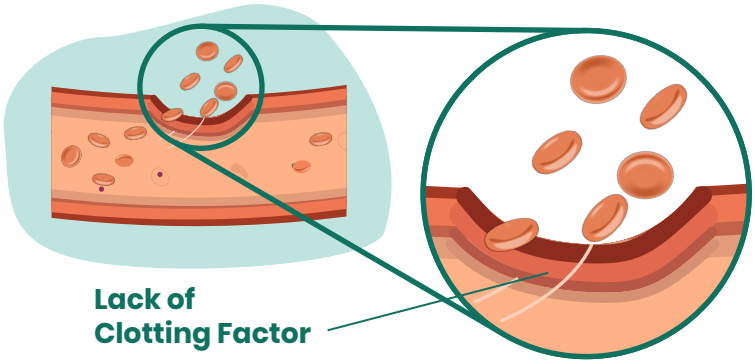
Blood Clots

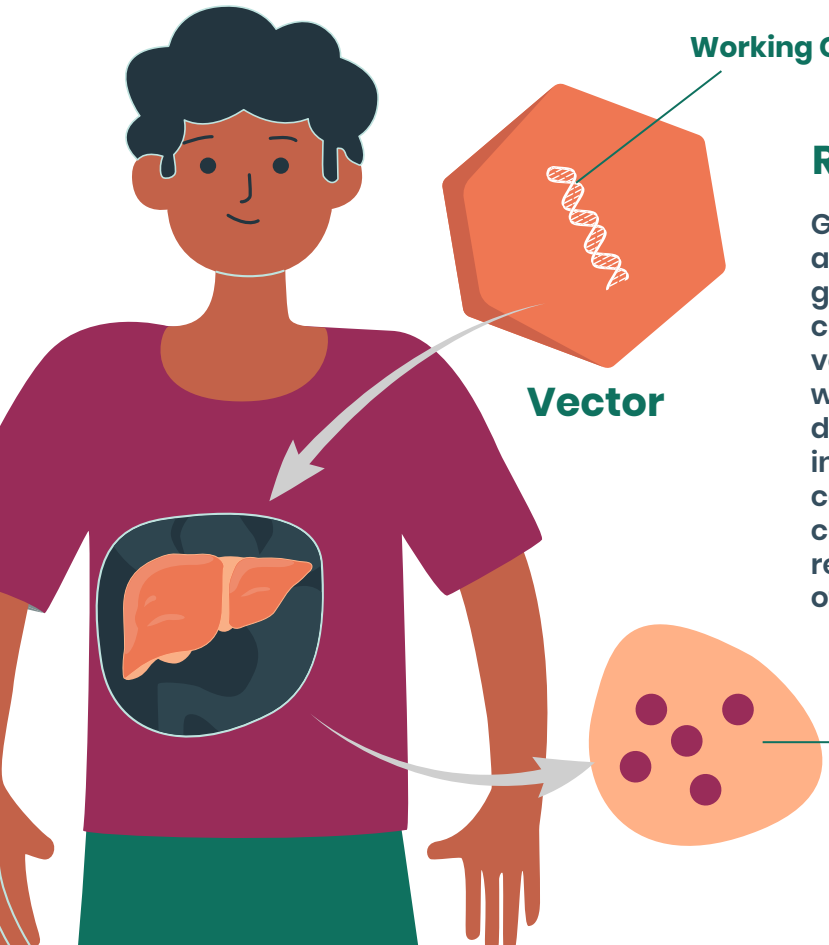
Blood clots form in response to a cut or other injury to a blood vessel. Platelets in the bloodstream stick to the cut edges of the ruptured vessels. Once the platelet plug is formed, proteins called clotting factors create a net-like mesh called fibrin that reinforces the plug and seals the wound.



Effect of Hemophilia

Hemophilia is a genetic disease that occurs when the genes that are vital to creating clotting factors are faulty. This results in prolonged external or internal bleeding in the body. The most common types of the disease—hemophilia A and hemophilia B—are caused by deficiencies of clotting factors VIII and IX.





Role of Gene Therapy

Gene therapy aims to be a one time administration that delivers working genes into the liver cells that are in charge of producing clotting factors. A vector, which is often derived from viruses with all viral genes removed, is used to deliver the new genes via infusion. By introducing the functional gene, the liver cells can produce functionally active clotting factors in the bloodstream, resulting in less bleeds and better control of hemophilia types A and B.

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