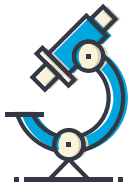
Hemophilia Dictionary

A REFERENCE ON HEMOPHILIA AND OTHER RARE BLEEDING DISORDERS

PLATELET



CARRIER

RECOMBINANT

aPTT

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acquired hemophilia

Hemophilia that is not passed down through the genes; it is caused by antibodies that the body develops against one's own clotting factors VIII or IX.

AIDS

(Acquired Immunodeficiency Syndrome): A disease caused by **HIV (Human Immunodeficiency Virus)**, which attacks and weakens the body's immune system.

antibodies

Proteins in the blood that attack substances that the body thinks present a danger. Antibodies that attack replacement therapies for hemophilia are called **inhibitors**.

aPTT

(Activated partial thromboplastin time): A blood test that looks at how long it takes for blood to clot. It can help tell if a person has bleeding or clotting problems.

baseline

A beginning point that is used to compare; it is sometimes used when measuring factor levels in the blood.

BU

(Bethesda Unit): A laboratory measurement of an **inhibitor**. Values above 5 are considered high; the inhibitor is powerful and weakens the effect of **factor product**.

bleed

Refers to an episode of bleeding (hemorrhage) which is leakage of blood out of the blood vessel, which can occur within the body or on its surface.

bleeding disorder

A chronic health condition in which the blood does not clot properly, resulting in excessive or lengthy bleeding.

blood clot

A thick clump or mass of blood.

breakthrough bleed

Bleeding that still occurs while on prophylactic treatment.

blood product

The part of donated blood that is used to treat hemophilia or other bleeding disorders. Examples of blood products include whole blood, packed red blood cells, fresh-frozen plasma, platelets, and **cryoprecipitate**.

bypassing agent

A product that contains one or more clotting factors to work around clotting **inhibitors**.

carrier

A person who has the gene for a condition and can pass it on to offspring, but does not necessarily display the symptoms. In hemophilia, a carrier is a female with an abnormal X chromosome carrying the hemophilia gene.

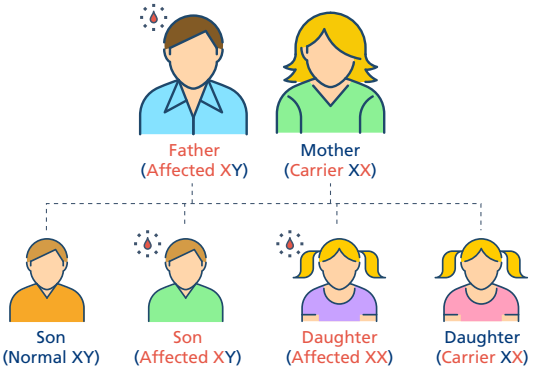
asymptomatic carrier

A carrier without symptoms of a condition.

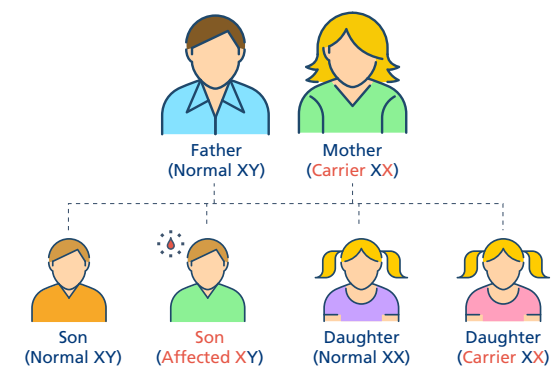
symptomatic carrier

A carrier with symptoms of a condition. In hemophilia, a symptomatic carrier has low factor levels and displays bleeding symptoms.

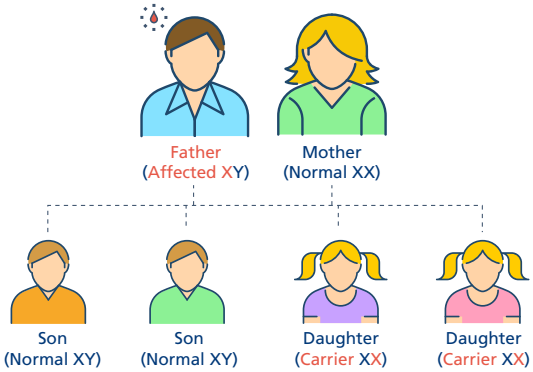
Father has the disease, Mother is a carrier



Mother is a carrier for disease



Father has the disease



CVAD

(Central venous access device): A device surgically implanted in the vein for easier access when infusing factor products.

chromosomes

Structures in the cell's nucleus that contain genetic information in the form of DNA.

clotting

The series of events by which the blood forms a clot to stop a bleed.

FAST FACT

Hemophilia A affects

1 in 5,000

male births

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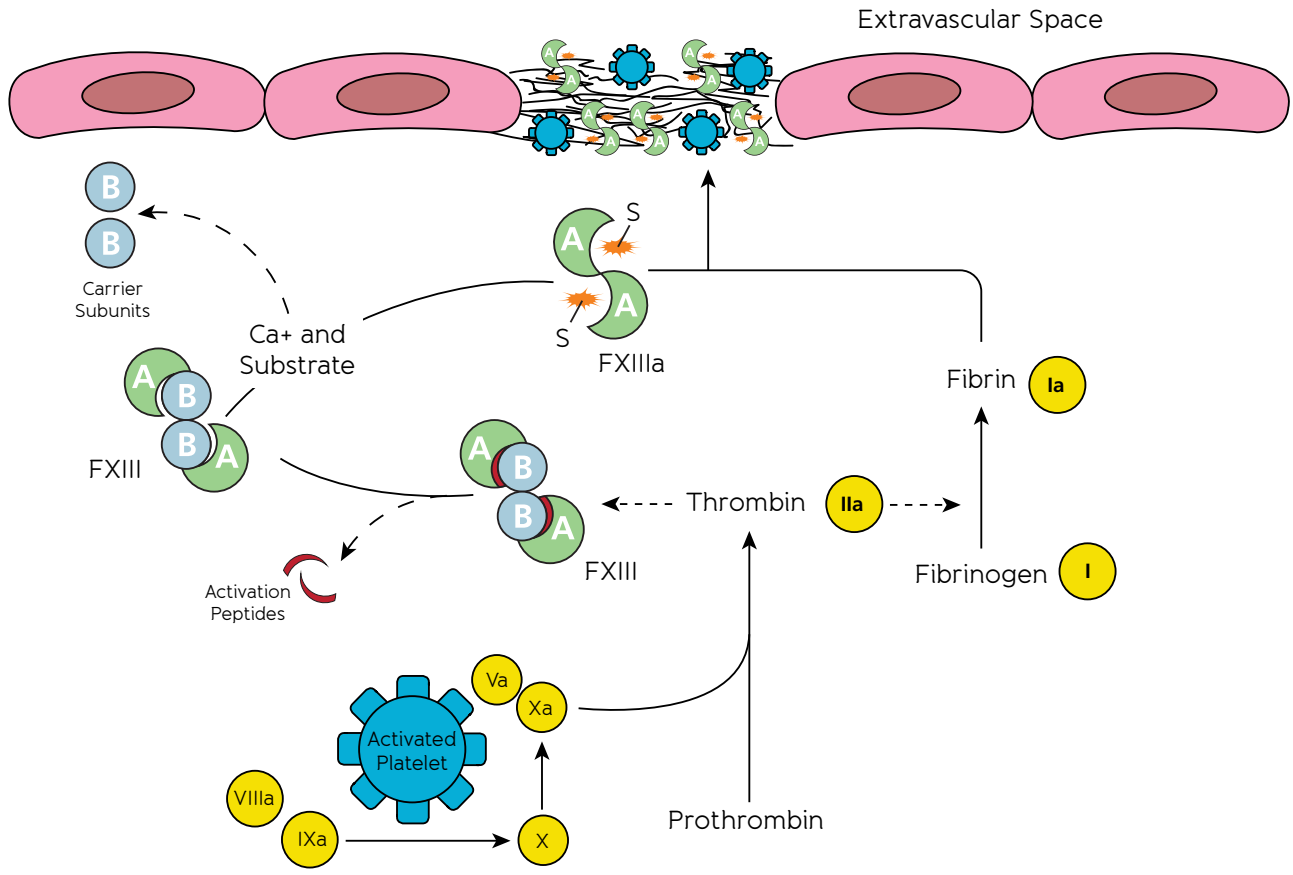
clotting cascade

A series of steps that occur to form a clot, involving the clotting proteins and other substances.

THE CLOTTING PROCESS



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clotting factors

Proteins in the blood that act in sequence to form a clot and stop bleeding.

coagulation

The process of forming a blood clot.

cryoprecipitate

A blood component made from plasma that was used to treat hemophilia A in the past.

FAST FACT

1 in every

500,000

people has factor VII deficiency

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DVT

(Deep vein thrombosis): A blood clot in a vein deep inside the body.

factor

Also known as factor product. Factor is a treatment that is infused to replace the body's missing clotting proteins. It is made from plasma or **recombinant** products.

factor assay

A lab test that determines the level of factor circulating in the body. The results are reported as a percentage of normal levels.

factor level

A measurement indicating how much clotting factor a person has in their blood. (Also known as factor activity or factor activity level.) People with hemophilia or other bleeding disorders have factor levels much lower than the standard.

factor XIII deficiency

A very rare and dangerous bleeding disorder, caused by a deficiency of factor XIII protein, which stabilizes the clot.

fresh frozen plasma

A part of whole blood used to treat hemophilia, mainly in the past.

gene

The basis of how characteristics and conditions get passed on from parents to their children. A gene is made up of DNA.

gene therapy

A medical intervention in which a gene that doesn't function properly is replaced with a gene that does.

Glanzmann's thrombasthenia

A congenital bleeding disorder in which the platelets are missing a natural element called glycoprotein IIb/IIIa. Because of this, the **platelets** do not function properly.

half-life

The amount of time it takes for the factor activity level to drop by half after an infusion. This helps patients understand how long an infusion can last as effective coverage.

hemarthrosis

Bleeding into a joint.

hematologist

A doctor who specializes in blood diseases, including **bleeding disorders**.

hemoglobin

A protein in red blood cells that carries oxygen.

hemophilia

A bleeding disorder that occurs mostly in males, caused by low levels of factor VIII or factor IX. The disorder makes bleeding hard to control.

hemophilia A

A bleeding disorder caused by lack of factor VIII. It is sometimes called "classic hemophilia."

hemophilia B

A bleeding disorder caused by lack of factor IX. It is sometimes called "Christmas disease."

mild hemophilia

A factor VIII or IX level ranging from 5% up to 40% of normal blood levels.

moderate hemophilia

A factor VIII or IX level ranging from 1% up to 5% of normal blood levels.

severe hemophilia

A factor VIII or IX level below 1% of normal blood levels.

HTC

(Hemophilia treatment center): A place that provides specialty care for hemophilia patients. If you have a bleeding disorder, it is a good idea to locate and get familiar with your nearest HTC.

hemorrhage

Blood escaping the blood vessels either internally or on the surface of the body.

hemostasis

See **clotting**.

hepatitis

Inflammation of the liver. It can be caused by infection from several hepatitis viruses, including hepatitis A, B, or C.

hepatitis A

Inflammation of the liver caused by the hepatitis A virus. There is a vaccine to prevent it.

hepatitis B

Inflammation of the liver caused by the hepatitis B virus. There is a vaccine to prevent it.

hepatitis C

Inflammation of the liver caused by the hepatitis C virus. There is no vaccine to prevent it.

HIV

(Human Immunodeficiency Virus): The virus that causes **AIDS (Acquired Immunodeficiency Syndrome)**.

ITI

(Immune Tolerance Induction): A therapy for people with hemophilia with inhibitors, who are given **factor product** regularly over a period of time until the body is trained to recognize the treatment product without reacting to it. When ITI is successful, **inhibitors** disappear and the patient's response to factor products returns to normal.

infusion

Delivering **factor** directly into a vein.

FAST FACT

Only

1.5 per million

people are affected by acquired hemophilia each year

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inhibitor

In rare bleeding disorders, inhibitors are antibodies in the blood that react to infused factor and slow the clotting process. Also see **antibodies**.

inhibitor titer

Inhibitor levels in the blood, measured in **BU (Bethesda units)**. A "low titer" inhibitor is measured at less than 5 BU; a "high titer" inhibitor is measured at or more than 5 BU.

intracranial hemorrhage

Bleeding inside the skull.

FAST FACT

Between **15% to 40%** of people with hemophilia A and **1% to 6%** with hemophilia B develop inhibitors.[a]

[a]Based on previously treated patients.

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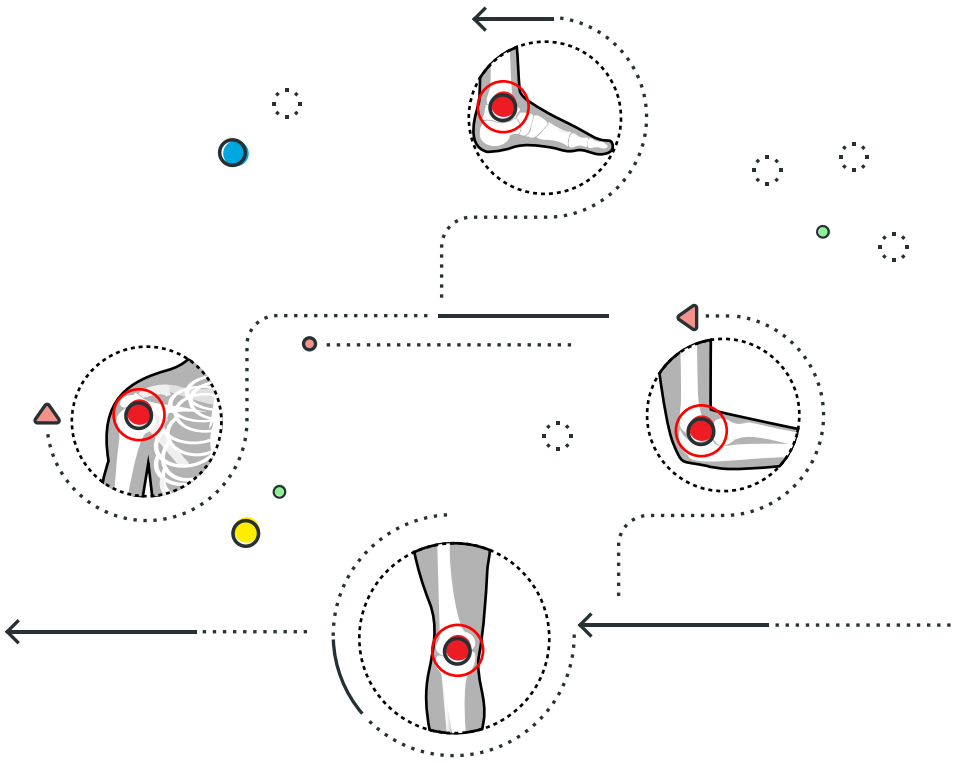
joint bleed

Bleeding in the joints, which is a common form of internal bleeding in people with hemophilia. It can occur without obvious injury or visible signs of bleeding. Bleeding that isn't treated quickly can damage the joint.



joint replacement

Using artificial components in a joint, such as the knee or elbow, to replace those that are damaged from wear and tear or chronic bleeds.

menorrhagia

Prolonged, heavy bleeding during menstruation; it can be a symptom of a bleeding disorder.

on-demand treatment

Factor that is infused as soon as a bleed occurs or is noticed; its purpose is to stop the bleed as quickly as possible.

parvovirus B19

An infectious virus that can potentially be passed on through plasma-derived blood products.

FAST FACT

Hemophilia B affects

1 in 25,000

male births

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PEGylation

Technology used to extend the **half-life** of a factor product.

plasma

The portion of the blood that contains proteins (including clotting factor proteins), immunoglobulin, and albumin.

platelet

Tiny cell particles in the blood that stick to an injured blood vessel, and to one another, to form a plug that helps stop bleeding.

platelet glycoproteins

Proteins that work together to connect platelets with one another.

platelet refractoriness

When platelet transfusions may not work as well as expected or at all in treating a bleeding episode, or preventing bleeding during a procedure.

port

A device that is surgically placed under the skin of the upper chest and attached to a tube that is inserted directly into a vein. It is used for infusing **factor product** or other medications.

prophylaxis

The regular infusion of **factor** in order to prevent bleeding.

prothrombin (factor II)

A protein needed to form a stable blood clot.

PT

(Prothrombin time): A blood test that measures the time it takes for the liquid portion of the blood (plasma) to clot.

rare bleeding disorder

A condition in which defects or low levels of **clotting factors** lead to lifelong bleeding problems.

recombinant

Genetically engineered factor product made without human blood or plasma.

FAST FACT

Nosebleeds are a common symptom of Glanzmann's thrombasthenia

synovectomy

A procedure performed to remove inflamed joint tissue (synovium) that is causing unacceptable pain or limiting a person's ability to function. This procedure can prevent further damage to the joint.

synovitis

Inflammation of the synovial membrane, which surrounds joints. It can be acute or caused by bleeding into the same joint.

target joint

A joint that has had repeated bleeds.

FAST FACT

>95% of people with FXIII deficiency are A-subunit deficient

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thrombin

A protein in the blood needed to form a stable clot.

thrombosis

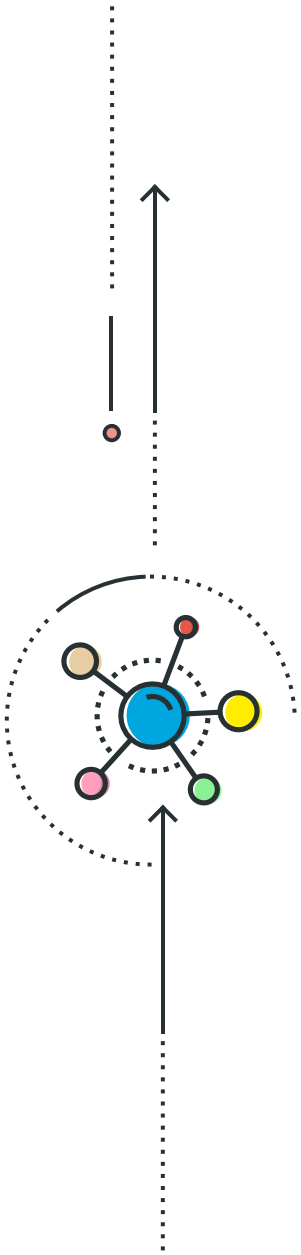
The formation of a blood clot within a blood vessel.

von Willebrand disease (vWD)

A bleeding disorder in which a patient is deficient in von Willebrand factor, causing clotting problems.

von Willebrand factor

The clotting protein that is deficient in vWD. There is a deficiency of von Willebrand factor or the existing factor does not work the way it should. Von Willebrand factor is also responsible for transporting factor VIII throughout the bloodstream.



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