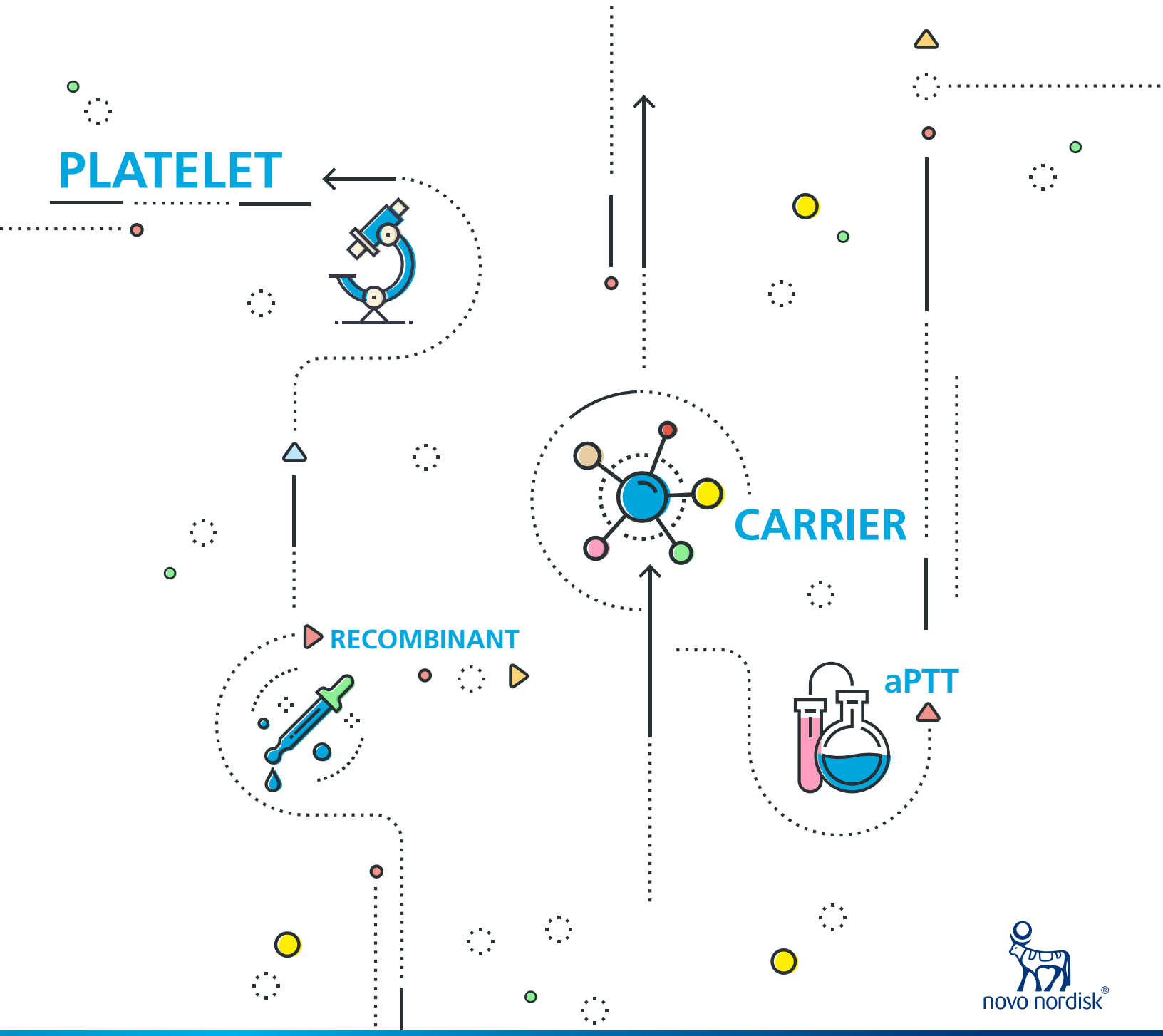
Hemophilia Dictionary

A REFERENCE ON HEMOPHILIA AND OTHER RARE BLEEDING DISORDERS



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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.

administration

The act of giving a treatment to a patient. It can also refer to the method of delivery, dose, or frequency.

AIDS

(acquired immunodeficiency syndrome): A disease caused by **HIV (human immunodeficiency virus)**, which attacks and weakens the body's immune system.

FAST FACT

1 in every

500,000

people has Factor VII deficiency

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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**.

anticoagulants

Substance that limits the body's ability to form a clot.

antithrombin

A protein in the blood that regulates blood clotting; it inactivates thrombin and other enzymes involved in the coagulation cascade.

aPTT

(Activated Partial Thromboplastin Time) A blood test that measures the time it takes for the liquid portion of the blood (plasma) to clot. An activator is added to speed up clotting time. This test is considered to be a more sensitive version of the Partial Thromboplastin Time (PTT) test.

baseline

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

bleed

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

bleed score

A measure of the risk of bleeding in people with hemophilia.

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.

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**.

breakthrough bleed

Bleeding that still occurs while on prophylactic treatment.

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**.

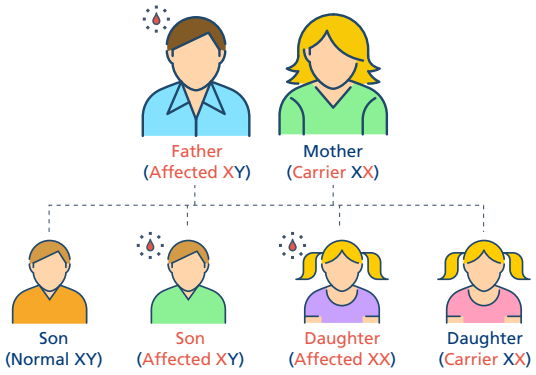
bypassing agent

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

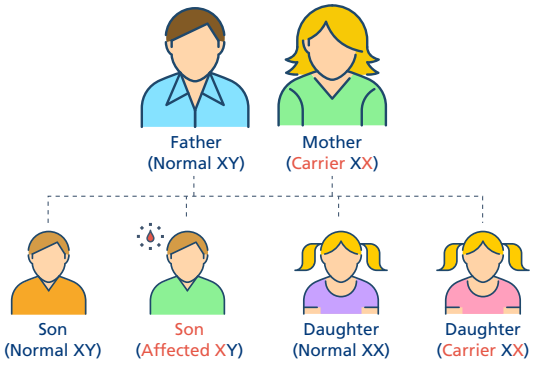
carrier

In hemophilia, a carrier is a female who has one X chromosome that carries the hemophilia gene and one X chromosome that does not.

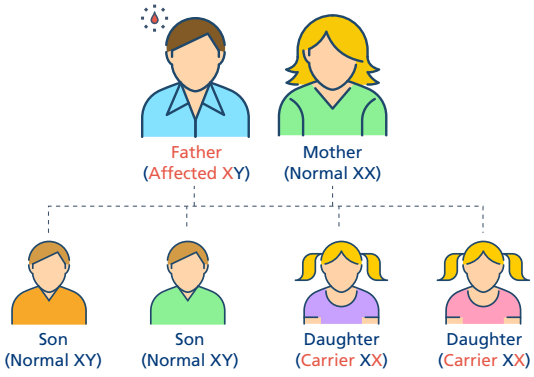
Father has the condition, Mother is a carrier



Mother is a carrier for condition



Father has the condition



Woman/Girl with hemophilia

A female with an X chromosome that carries the hemophilia gene and with factor levels below 40%.

Woman/Girl with mild hemophilia: A woman/girl with factor levels ranging from 5% up to 40% of normal blood levels.

Woman/Girl with moderate hemophilia: A woman/girl with factor levels ranging from 1% up to 5% of normal blood levels.

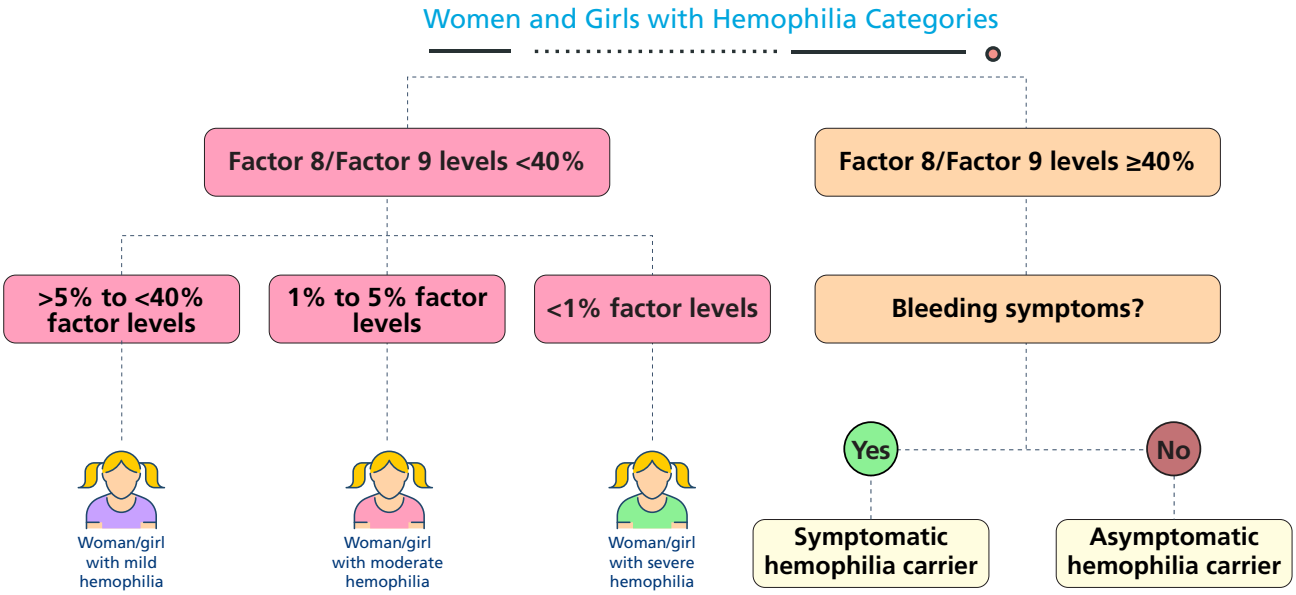
Woman/Girl with severe hemophilia: A woman/girl with factor levels below 1% of normal blood levels.

asymptomatic carrier

A carrier without symptoms of a condition. In hemophilia, an asymptomatic carrier has ≥40% factor levels and does not display bleeding symptoms.

symptomatic carrier

A carrier with symptoms of a condition. In hemophilia, a symptomatic carrier has ≥40% factor levels and displays bleeding symptoms.



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chromosomes

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

clotting (also known as coagulation)

A sequence of events that occurs to stop bleeding when a blood vessel becomes injured and bleeding occurs. People with hemophilia A do not produce enough clotting Factor VIII, and people with hemophilia B do not produce enough Factor IX for clots to form properly.

FAST FACT

Hemophilia A affects

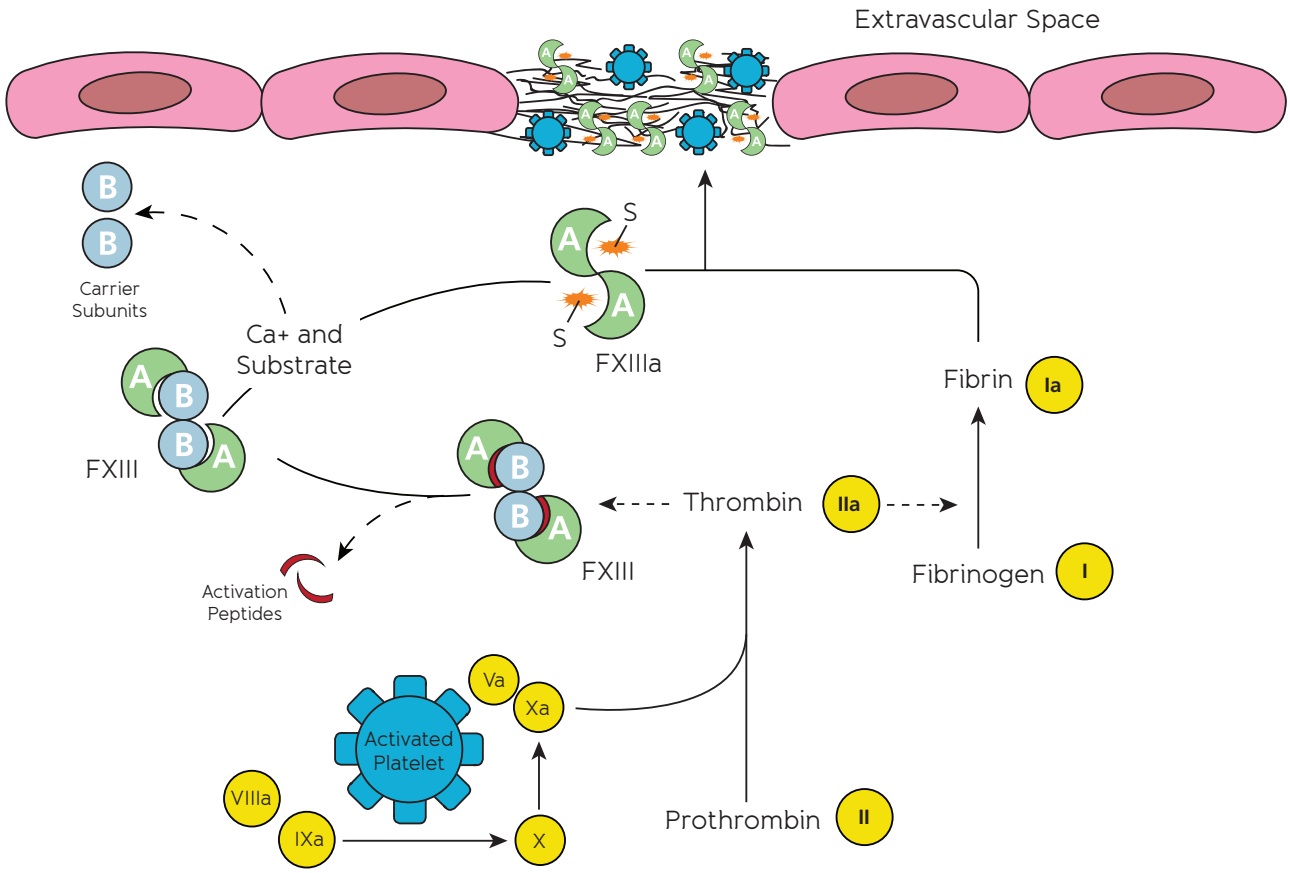
1 in 5,000

male births

clotting cascade

A series of steps that occurs to form a clot, involving the clotting proteins and other substances. Also known as coagulation cascade.

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 is also known as the coagulation cascade.

cryoprecipitate

A blood component made from plasma used to prevent or control bleeding in people whose own blood does not clot properly.

CVAD

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

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 replacement therapies

Treatments for hemophilia that increase the amount of factor in the body to levels that lead to better clotting, and therefore less bleeding; also called clotting factor replacement therapy.

Factor XIII deficiency

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

fibrin clot

A clot that is formed primarily of fibrin, which is a protein that is produced as a response to bleeding.

fresh frozen plasma

A part of whole blood that has been used to treat people with hemophilia.

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 treatment where new working genes are introduced into a person's cells to fight disease. In the case of hemophilia, the new genes give the body instructions on how to make factor. There are different kinds of gene therapy, including gene transfer, gene editing, and cell therapy.

Glanzmann thrombasthenia

A congenital bleeding disorder in which 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 people 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.

FAST FACT

Only

1.5 per million

people are affected by acquired hemophilia each year

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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.

hemorrhage

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

hemostasis

A process that ensures bleeding stops at the site of injury while normal blood flow continues to occur elsewhere in the body. 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

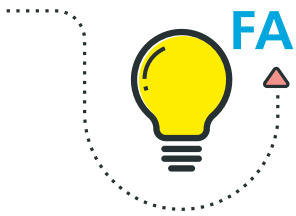
Inflammation of the liver caused by the hepatitis A virus. There is a vaccine to prevent it.

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.



FAST FACT

Nosebleeds are a common symptom of Glanzmann thrombasthenia

HIV

(human immunodeficiency virus): The virus that causes **AIDS (acquired immunodeficiency syndrome)**.

HTC

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

intravenous infusion

Delivering **factor** directly into a vein. Also known as an IV, or intravenous (within a vein).

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.

injection

Using a needle and syringe to insert fluid, such as medication, into the body.

intracranial hemorrhage

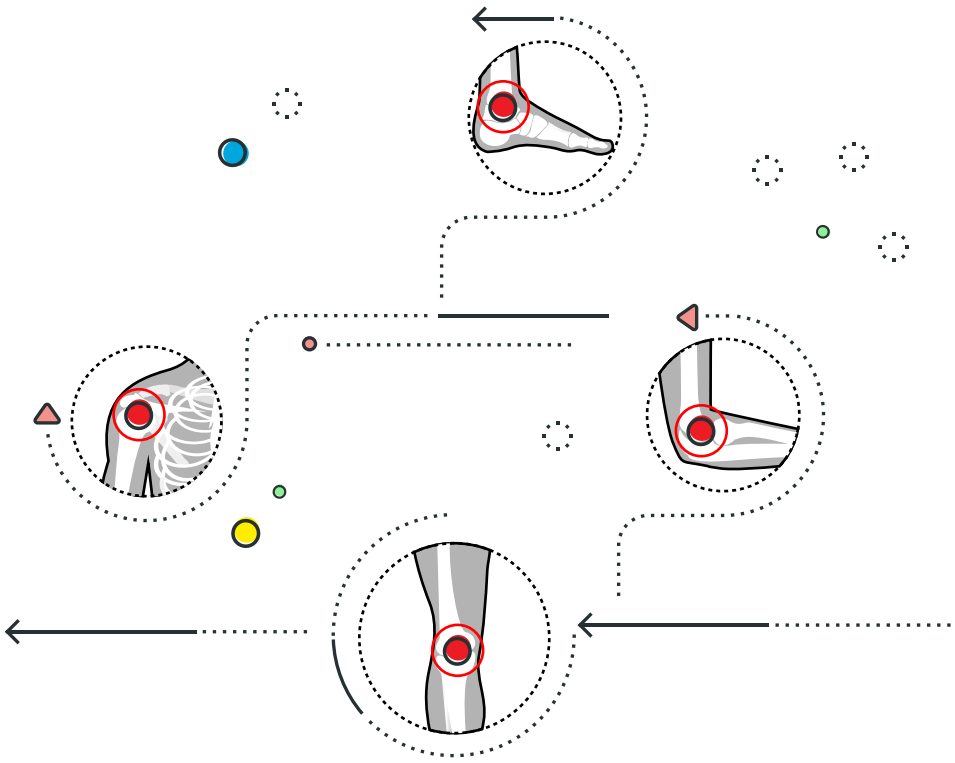
Bleeding inside the skull.

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.

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 is not 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.

monoclonal antibody

An antibody is a protein that binds to a target of interest and can block it from functioning. A monoclonal antibody is a man-made substance used to treat some diseases.

nonfactor treatment

A type of treatment for hemophilia that works independently of replacement factor administration to enhance coagulation or inhibit anticoagulant pathways.

FAST FACT

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

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.

PEGylation

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

plasma

The portion of the blood that contains proteins (including clotting factors, immunoglobulins, 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 do 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.

procoagulant

A protein that promotes formation of thrombin and fibrin to increase coagulation (clotting).

protein

A complex molecule that can have many functions in the body. Proteins play a key role in blood coagulation.

prothrombin (Factor II)

A protein that gets converted into thrombin and is 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.

rebalancing therapies

Treatment focused on the balance of procoagulant and anticoagulant proteins within the coagulation cascade.

recombinant

Genetically engineered factor product made without human blood or plasma.

RNAi

Also known as RNA interference, it is a process in which RNA is used to interfere with gene expression so that a specific protein is not made.

subcutaneous administration

An injection delivered under the skin.

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.

thrombin

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

thrombosis

The formation of a blood clot in the vein or artery that inhibits blood flow.

tissue factor

A protein that plays a key role in activating blood coagulation.

tissue factor pathway inhibitor

An anticoagulant protein that limits the production of thrombin.

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. Von Willebrand factor binds to factor VIII, another clotting protein, and platelets in blood vessel walls.

FAST FACT

Hemophilia B affects

1 in 25,000

male births

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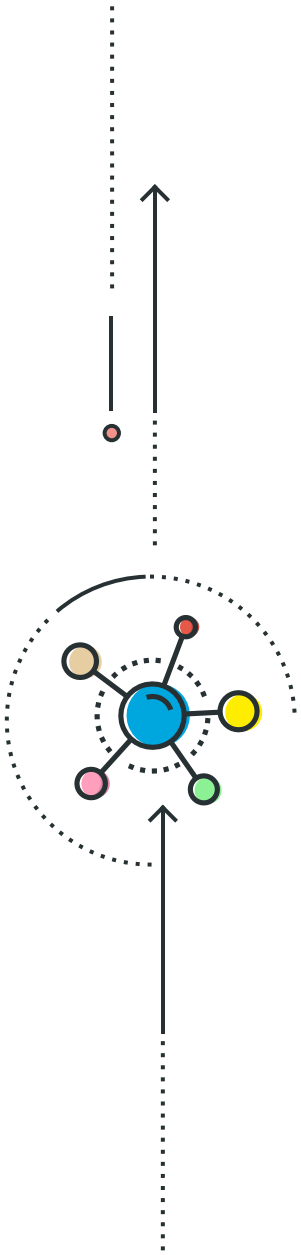

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