

New and Developing Treatment Options for Hemophilia

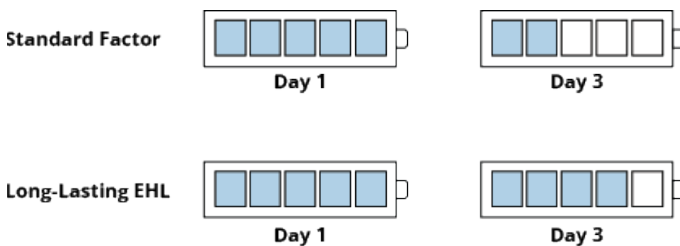
Hemophilia treatment is changing fast and there are now many choices. This guide gives facts to help you talk with your care team. All of these treatment options aim to prevent bleeds and provide protection. The right choice depends on your lifestyle, your body, and your goals, and can change over time.



What are the current treatment options and how do they work?

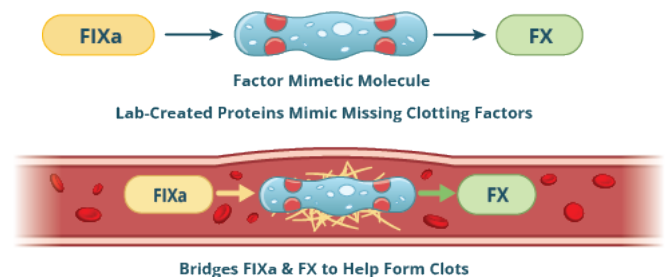
ULTRA-EXTENDED HALF-LIFE FACTOR THERAPY

This treatment uses a longer-lasting form of factor VIII. It works by staying active in the body for more time, which helps keep factor levels higher between doses.



FACTOR MIMETIC THERAPY

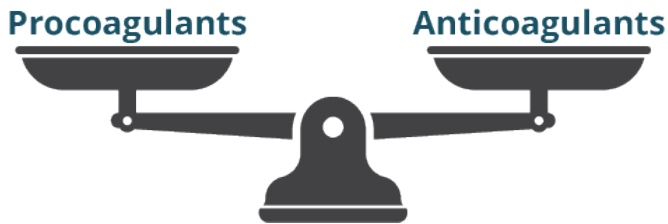
This treatment is only for hemophilia A. This treatment does not replace factor VIII but acts like factor VIII inside the clotting process.



¹ This guide was created in 2026 by people living with hemophilia, their caregivers, and their healthcare providers in Canada with partners from Queen's University and the Center of Excellence on Partnership with the Patients and the Public. Some details and treatment options may be different where you live, so please verify with your care team.

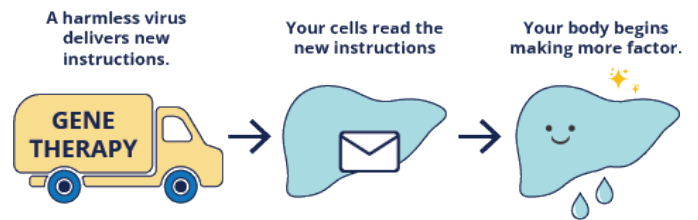
REBALANCING AGENTS

These medicines block the body's natural anti-clot proteins. By reducing these proteins, they help the blood clot more easily for people with hemophilia A or B.



GENE THERAPY

This is a one-time treatment given through an intravenous injection. It works by giving the liver a working copy of the missing gene so the body can make its own factor over time.



Complementary Information:



World Federation of Hemophilia (WFH)
<https://wfh.org/>



Canadian Hemophilia Society
<https://www.hemophilia.ca/>

Contact your local clinic or health care center for more information.



Treatment Options Comparison Table

Key Questions	Extended half-life factor therapy	Factor mimetic	Ultra-extended half-life factor therapies	Rebalancing agents	Gene therapy
Who can use it?	Children and adults with hemophilia A and B, without inhibitors	Children and adults with hemophilia A, with and without inhibitors	Children and adults with hemophilia A, without inhibitors	Being studied in teens and adults with Hemophilia A and B as of 2025	Approved for Hemophilia B patients who require prophylactic treatment
How well does it work?	Good bleed protection.	Good bleed protection	Keeps factor VIII levels high for most of the week. Good bleed protection	Good bleed protection	Most patients reach normal or near-normal factor levels that last for many years
What it is?	Recombinant factor VIII or IX	Non-factor medicine	Modified factor VIII that lasts longer in the body	Medicines that encourage clotting by reducing anti-clot proteins	A one-time intravenous treatment that delivers a working gene
Is it for treatment or prevention?	Prevention (prophylaxis) and treatment	Prevention (prophylaxis)	Prevention (prophylaxis) and treatment	Prevention (prophylaxis)	Prevention (prophylaxis)
How is it given?	Intravenous (into the vein) infusion at home	Subcutaneous (under the skin) injection at home	Intravenous (into the vein) infusion at home	Subcutaneous (under the skin) injection at home	Intravenous infusion in a hospital
How often?	Once to twice per week	Weekly to monthly	Usually once a week	Daily to monthly	Once in a lifetime
Side effects	Headache, fever, mild infusion reactions. Very rare inhibitor development	Mild skin reactions, headache. Rare serious clots if mixed with certain medicines	Headache, fever, mild infusion reactions. Very rare inhibitor development	Possible liver problems, rare clot risk	Liver inflammation, nausea, tiredness. May not work forever

Treatment Options Comparison Table

Key Questions	Extended half-life factor therapy	Factor mimetic	Ultra-extended half-life factor therapies	Rebalancing agents	Gene therapy
Benefits	Good bleed protection. Less frequent doses compared to standard half-life factor	No IV access needed. Works even with inhibitors	Only one weekly infusion. High factor levels between doses	Works for hemophilia A and B, with or without inhibitors. Monthly dosing possible	No regular needles. Long-lasting bleed protection
Limits	Requires IV access	Only for hemophilia A	Requires IV access. Only for hemophilia A.	Not yet fully approved. Long-term safety still being studied	Must have healthy liver. Not for kids. Levels may fall over time. One time only treatment. Only for Hemophilia B.
Burden of care	Medium. IV access needed.	Low. No IV. Easy to store	Medium. IV access needed but fewer infusions	Low. Injection at home	Busy treatment period for 6-12 months, then annual monitoring
Storage and travel	Must store factor at home or carry it when travelling. May need cooling.	Easy to store. Good for travel	Must store factor at home or carry it when travelling. May need cooling	Easy to store and portable	No ongoing medicine to carry
Common trade names	FVIII : Jivi, Eloctate, Adynovate; FIX: Rebinyn, Alprolix	Hemlibra	Altuviiiio	Qfitlia (Fitusiran), Alhemo (conclizumab), Hymvapzi (marstacimab)	Hemgenix

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