

Comparison of Prophylaxis Options in Hemophilia (2025)



Treatment Type	Who May Benefit	How It's Given	How Often	Monitoring Needs	Lifestyle Benefits/Considerations
Factor Replacement Therapy (Standard half-life) Recombinant factor VIII (e.g., Afstyla®, Advate®, Kovaltry®, Novoeight®, Nuwiq®, Xyntha Solofuse®; or IX (e.g., BeneFIX®, Ixinity®, Rixubis®))	Replaces the missing factor in children and adults (products specific for hemophilia A or B depending on product)	Intravenous (IV) infusion into a vein	Usually 2-4 times per week	Periodic factor level checks; bleeding log	• Longest history of evidence of safety and efficacy • Allows a more active lifestyle versus no prophylaxis • Requires venous access • Impact on schedule: time-consuming
Factor Replacement Therapy (Extended half-life) Extended half-life (EHL) factor VIII (e.g., Altuviiro®, Elocate®, Esperoct®, Jivi®); or IX (e.g., Alprolix®, Idelvion®, Rebinyn®)	Replaces the missing factor in children and adults (products specific for hemophilia A or B depending on product)	IV infusion into a vein	Usually 1-2 times per week	Periodic factor level checks; bleeding log	• Evidence of safety and efficacy for over a decade • Fewer infusions versus the standard half-life agents • More flexibility for travel, school, or work than standard half-life agents • Requires venous access • Impact on schedule: time-consuming

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Non-Factor Therapies (Subcutaneous [SQ] Injection)					
Emicizumab (Hemlibra®)	Mimics factor VIII activity (for hemophilia A, with or without inhibitors; in patients who are newborns and older)	Under the skin	Once weekly, every 2 weeks, or every 4 weeks	No routine lab monitoring; periodic clinical follow-up	• Longest history of safety and efficacy among non-factor therapies • Fewer needle sticks • Steady protection • No IV infusions • Impact on schedule: flexible dosing • Self-injection with vial and syringe • Can be out of the refrigerator for up to 7 days • Cannot be combined with activated prothrombin complex concentrate (aPCC) except under medical guidance due to boxed warning of thrombotic microangiopathy and thromboembolism
Concizumab (Alhemo®)	Blocks tissue factor pathway inhibitor (TFPI) to promote clotting (for hemophilia A and B, with or without inhibitors; in patients age 12 and older)	Under the skin	Once daily	Ongoing follow-up for bleeding control and injection reactions	• Found safe and effective in clinical trials • Steady protection • No IV infusions • Impact on schedule: consistent daily routine with easy self-injection • Adjustable dose, pre-filled pen device • Once used, can be out of the refrigerator for up to 4 weeks

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Non-Factor Therapies (Cont.) (Subcutaneous [SQ] Injection)					
Marstacimab (Hypmavzi™)	Also targets TFPI to help restore normal clotting (for hemophilia A and B, without inhibitors in patients age 12 and older)	Under the skin	Once weekly	Ongoing follow-up for bleeding control and injection reactions	• Found safe and effective in clinical trials • Steady protection • No IV infusions • Impact on schedule: weekly, easy self-injection • Fixed-dose, pre-filled pen device • Can be out of the refrigerator for up to 7 days
Fitusiran (Qfitlia™)	Lowers antithrombin via RNA interference to enhance clotting (for hemophilia A and B, with or without inhibitors, in patients age 12 and older)	Under the skin	Once every 2 months	Regular blood tests for antithrombin and liver function; signs and symptoms of gallbladder disease: initial testing frequency higher than during maintenance dosing	• Found safe and effective in clinical trials • Steady protection • No IV infusions • Impact on schedule: every-other-month, easy self-injection • Fixed-dose, pre-filled pen device • Can be out of the refrigerator for up to 3 months • Has a boxed warning for thrombotic events and acute and recurrent gallbladder disease

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Gene Therapy (e.g., Etranacogene dezaparvovec [Hemgenix®] for hemophilia B; valoctocogene roxaparvovec [Roctavian®] for hemophilia A)	Provides a working gene to tell the body to make its own missing factor for certain adults with hemophilia A or B depending on product	One-time IV infusion at a treatment center	One-time treatment	Regular liver enzyme and factor level checks for months afterward, then long-term monitoring for durability	- May reduce or eliminate the need for ongoing prophylaxis for years - Impact on schedule: initial monitoring requirements following administration are time-consuming for several months
Other Bypassing Agents (For patients with inhibitors) (e.g., aPCC [FEIBA®], recombinant factor VIIa [NovoSeven®, Sevenfact®])	Helps form clots without replacing the missing factor in patients with hemophilia A and B with inhibitors	IV infusion	Varies (prophylactic or on-demand)	Periodic specialist monitoring	- Used mainly for inhibitor management - Dosing individualized

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