

Wyoming Drug Utilization Review

Recently Approved Medications for Hemophilia A and Hemophilia B

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Hemophilia used to indicate a shortened life span for individuals with the condition. With innovative technology, that is no longer the case.¹ Hemophilia affects about 1 in 10,000 live births, with a higher prevalence in hemophilia A, which accounts for about 80% to 85% of those affected compared to hemophilia B. It is more common in males due to an X-linked inheritance pattern.¹ Hemophilia A is caused by a congenital deficit of clotting factor VIII, leading to protracted and excessive bleeding.² Hemophilia B is caused by a defect in the F9 gene, leading to insufficient clotting factor IX.³ Hemophilia C is a deficiency in Factor XI, but is very rare.¹ Increasing technology has allowed for an expansion of the market with new agents for hemophilia A and B.

Novel therapies in hemophilia include gene therapy and the use of monoclonal antibodies.^{1,4} Among monoclonal antibodies (mAbs), there are two newer tissue factor pathway inhibitors (TFPI) antagonists^{5,6}, marstacimab-hncq (Hymoviz)⁵ and concizumab-mtci (Alhemo).⁶ Hymoviz is indicated for routine prophylaxis for the reduction or prevention of bleeding episodes in patients 12 years of age or older with hemophilia A without factor VIII inhibitors or hemophilia B without factor IX inhibitors.⁵ It works on the Kunitz domain 2 (K2) of TFPI and is subcutaneously administered once weekly.⁵ For all dosing information, refer to the table below. Alhemo is indicated for routine prophylaxis to reduce or prevent bleeding episodes in patients older than 12 years who have hemophilia A with FVIII inhibitors or hemophilia B with FIX inhibitors.⁶ It is administered once daily subcutaneously. Four weeks after Alhemo is initiated, drug plasma concentration should be measured using Enzyme-Linked Immunosorbent Assay (ELISA) prior to the administration of the next scheduled dose (see table below).⁶ Additional gene therapy medications for hemophilia are currently in trial phases.⁴

Gene therapy advances include valoctocogene roxaparvovec-rvox (Roctavian)⁷, etranacogene dezaparvovec (Hemgenix)⁸, and fidanacogene elaparvovec (Beqvez).⁹ Roctavian is indicated for adults with severe hemophilia A without pre-existing antibodies to adeno-associated virus serotype 5 (AAV5).⁷ Roctavian is an AAV5-based gene therapy that works by introducing a functional copy of human coagulation factor VIII by using a transgene encoding the B-domain deleted SQ form of VIII (hFVIII-SQ). This transcription occurs within the liver. Roctavian utilizes a one-time single-dose intravenous (IV) infusion.⁷ Hemgenix is indicated for adults with hemophilia B who are currently using Factor IX prophylaxis therapy, or currently have or have historically had a life-threatening hemorrhage, or have had repeated serious spontaneous bleeding episodes.⁸ It is also an AAV5-based gene therapy but works by delivering a copy of a gene encoding the Padua variant of human coagulation Factor IX (hFIX-Padua). Hemgenix, like Roctavian, is a one-time IV infusion.⁸ Beqvez is indicated for adults with moderate to severe hemophilia B who are currently

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using factor IX prophylaxis therapy, or have a current or historical life-threatening hemorrhage, or repeated serious spontaneous bleeding episodes, and do not have neutralizing antibodies to the adeno-associated virus serotype Rh74var (AAVRh74var) capsid.⁹ It is a gene therapy that introduces a functional copy of the factor IX gene that encodes for a high-activity FIX variant (FIX-R338L, hFIX Padua) through the AAVRh74var capsid within hepatocytes. Beqvez is a one-time single-dose IV infusion.⁹

A newer medication on the market, approved in 2025¹⁰, is neither a monoclonal antibody nor a gene therapy. Fitusiran (Qfitlia) is indicated for routine prophylaxis to prevent or reduce bleeding episodes in patients 12 years of age or older with hemophilia A or B with or without factors VIII or IX inhibitors.¹⁰ It is a double-stranded siRNA that works by causing the degradation of antithrombin messenger RNA, causing a reduction in plasma antithrombin levels. Qfitlia is a subcutaneous injection given once every 2 months.¹⁰

Medications	Dosing
Hypavzi (marstacimab-hncq)	Loading: 300 mg (two 150 mg injections) subcutaneous injection ⁵ Maintenance: 150 mg once weekly subcutaneously beginning one week after loading dose ⁵
Alhemo (concizumab-mtci)	Day 1: Loading: 1 mg/kg ⁶ Day 2: 0.2 mg/kg once daily subcutaneously until individualization of maintenance dose Continue 0.2 mg/kg once daily subcutaneously for 4 weeks Individualized dosing based on plasma concentration of Alhemo: • >200 ng/mL: adjust to 0.25 mg/kg • 200 to 4,000 ng/mL: continue 0.2 mg/kg • >4,000 ng/mL: adjust to 0.15 mg/kg⁶
Roctavian (valoctocogene roxaparvovec-rvox)	6×10^{13} vector genomes per kilogram (vg/kg) in a single IV infusion at a rate of 1 mL/min ⁷
Hemgenix (etranacogene dezaparvovec)	2×10^{13} genome copies per kg (gc/kg) at an infusion rate of 8 mL/min ⁸
Beqvez (fidanacogene elaparvovec)	5×10^{11} vg/kg with adjusted body weight being used in individuals with a BMI >30 kg/m ² ⁹ Given as a final volume of 200 mL over 60 minutes ⁹
Qfitlia (fitusiran)	Starting dose: 50 mg subcutaneously every two months ¹⁰ Dose is changed based on Antithrombin Activity (see package insert for more information) ¹⁰

Table 1: Dosing of New Hemophilia A and Hemophilia B Medications

The therapeutic landscape for hemophilia has evolved substantially. New technology, including mAbs, gene therapy, and the utilization of siRNA has contributed greatly to the lifespan of hemophilia patients. Additional medications, especially within gene therapy, are undergoing clinical trials showing further expansion. Emerging technologies are anticipated to further advance care, underscoring the continued paradigm shift in the management of hemophilia.

References

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6. Alhemo (concizumab-mtci) [package insert]. Plainsboro (NJ): Novo Nordisk Inc; 2024 December.
7. Roctavian (valoctocogene roxaparvovec-rvox) [package insert]. Novato (CA): BioMarin Pharmaceutical Inc; 2023 June.
8. Hemgenix (etranacogene dezaparovec-drlb) [package insert]. King of Prussia (PA): CSL Behring LLC; 2022 November.
9. Beqvez (fidanacogene elaparvovec-dzkt) [package insert]. New York (NY): Pfizer; 2024 April.
10. Qfitlia (fitusiran) [package insert]. Cambridge (MA): Sanofi; 2025 March.

The P&T Committee met for its quarterly business meeting on May 14, 2026.

Highlights of this meeting include:

The new PBA system went live April 15, 2026. Electronic prior authorization is now live.

The Department of Health continues to work on the Rural Health Transformation program. A total of \$205 million was awarded for the first year. Funds must be allocated by October. Additional information can be found at <https://health.wyo.gov/admin/rural-health-transformation-program/>.

Non-preferred medications for chronic spontaneous urticaria will require a 56-day trial of a preferred medication.

Lerechol, Icotyde and Nereus were limited to indication and referred to the Department of Health for cost analysis and PDL placement.

Yuviwel, Kygevi, Zycubo and Loargys were limited to indication.

All prior authorization criteria are open for public comment. Comments should be sent by email to alewis13@uwyo.edu by June 15, 2026.

The next P&T Committee meeting will be held August 20, 2026 in Cheyenne. An agenda will be posted approximately two weeks prior to the meeting.

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