

PRIOR AUTHORIZATION POLICY

- POLICY:** Hemophilia – Non-Factor Routine Prophylaxis Products – Qfitlia Prior Authorization Policy
- Qfitlia™ (fitusiran subcutaneous injection – Genzyme/Sanofi)

REVIEW DATE: 06/11/2025

OVERVIEW

Qfitlia, an antithrombin-directed small interfering ribonucleic acid, is indicated for the routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and pediatric patients ≥ 12 years of age with hemophilia A or B with or without Factor VIII or Factor IX inhibitors.¹

Qfitlia is given by subcutaneous (SC) injection only.¹ The initial dose is 50 mg SC once every 2 months (Q2M). Monitor antithrombin activity utilizing an FDA-cleared test. Maintain antithrombin activity between 15% to 35% by adjusting the dose and/or frequency of administration. Other dose regimens include: 50 mg SC once monthly (QM); 20 mg SC Q2M; 20 mg SC QM; 10 mg SC Q2M; and 10 mg SC QM. Qfitlia may be given by the patient and/or caregiver after proper training. In pediatric patients 12 to 17 years of age, Qfitlia should be given by or under supervision of an adult.

Disease Overview

Hemophilia A and B are genetic bleeding disorders caused by a dysfunction or deficiency of coagulation Factor VIII and Factor IX, respectively.²⁻⁷ Because hemophilia is an X-linked condition, males are primarily impacted. Patients who have these types of hemophilias are not able to properly form clots in the blood and may bleed for a longer time than normal following injury or surgery. Patients may also experience spontaneous bleeding in muscles, joints, and organs. Bleeds may be life-threatening. A main morbidity is hemophilic arthropathy, which limits mobility. It is estimated that 33,000 males are living with hemophilia in the US; hemophilia A accounts for around 80% of the cases (approximately 26,400 patients) and hemophilia B comprises 20% of cases (around 6,600 patients). Hemophilias are often classified as mild, moderate, or severe based on reduced Factor VIII or IX levels. Approximately 50% and 30% of patients with hemophilia A and hemophilia B, respectively, have severe disease. The formation of inhibitors (antibodies) to factor products is a challenging complication as it causes Factor VIII and Factor IX therapies to be ineffective, which increases bleeding frequency and severity. Inhibitors develop in around 30% and 10% of patients with severe hemophilia A and hemophilia B, respectively.

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of Qfitlia. All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Qfitlia as well as the monitoring required for adverse events and long-term efficacy, approval requires Qfitlia to be prescribed by or in consultation with a hemophilia specialist.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Qfitlia is recommended in those who meet one of the following criteria:

FDA-Approved Indications

1. Hemophilia A without Factor VIII Inhibitors. Approve for 1 year if the patient meets ONE of the following (A or B):

- A) Initial Therapy.** Approve if the patient meets ALL of the following (i, ii, iii, iv, v, and vi):
- i.** Patient is ≥ 12 years of age; AND
 - ii.** Patient is using Qfitlia for routine prophylaxis to prevent or reduce the frequency of bleeding episodes; AND
 - iii.** Patient has moderately severe to severe hemophilia A as evidenced by a baseline (without Factor VIII replacement therapy) Factor VIII level of $\leq 2\%$; AND
 - iv.** Patient meets ONE of the following (a or b):
 - a)** Patient meets BOTH of the following [(1) and (2)]:
 - (1) Factor VIII inhibitor titer testing has been performed within the past 30 days; AND
 - (2) Patient does not have a positive test for Factor VIII inhibitors of ≥ 1.0 Bethesda units/mL; OR
 - b)** Patient has not received Factor VIII therapy in the past; AND
 - v.** According to the prescriber, prophylactic use of Factor VIII products will be discontinued no later than 7 days following the initial Qfitlia dose; AND
Note: Use of Factor VIII products for the treatment of breakthrough bleeding is permitted.
 - vi.** The medication is prescribed by or in consultation with a hemophilia specialist; OR
- B) Patient is Currently Receiving Qfitlia.** Approve if the patient meets ALL of the following (i, ii, iii, and iv):
- i.** Patient is using Qfitlia for routine prophylaxis to prevent or reduce the frequency of bleeding episodes; AND
 - ii.** According to the prescriber, prophylactic use of Factor VIII products will not occur while receiving Qfitlia; AND
Note: Use of Factor VIII products for the treatment of breakthrough bleeding is permitted.
 - iii.** The medication is prescribed by or in consultation with a hemophilia specialist; AND
 - iv.** According to the prescriber, patient experienced a beneficial response to therapy.
Note: Examples of a beneficial response to therapy include a reduction in bleeding events, in the severity of bleeding episodes, in the number of bleeding events that required treatment, and/or in the number of spontaneous bleeds.

2. Hemophilia A with Factor VIII Inhibitors. Approve for 1 year if the patient meets ONE of the following (A or B):

- A) Initial Therapy.** Approve if the patient meets ALL of the following (i, ii, iii, iv, v, and vi):
- i.** Patient is ≥ 12 years of age; AND
 - ii.** Patient is using Qfitlia for routine prophylaxis to prevent or reduce the frequency of bleeding episodes; AND
 - iii.** Patient meets BOTH of the following (a and b):
 - a)** Factor VIII inhibitor titer testing has been performed within the past 30 days; AND
 - b)** Patient has a positive test for Factor VIII inhibitors of ≥ 0.6 Bethesda units/mL; AND
 - iv.** According to the prescriber, prophylactic use of bypassing agents will be discontinued no later than 7 days following the initial Qfitlia dose; AND
Note: Use of bypassing agents for the treatment of breakthrough bleeding is permitted. Examples of bypassing agents include NovoSeven RT (coagulation Factor VIIa [recombinant] intravenous infusion), Sevenfact (Factor VIIa [recombinant]-jnew intravenous infusion), and FEIBA (anti-inhibitor coagulant complex intravenous infusion).
 - v.** Patient is not undergoing immune tolerance induction therapy; AND
 - vi.** The medication is prescribed by or in consultation with a hemophilia specialist; OR
- B) Patient is Currently Receiving Qfitlia.** Approve if the patient meets ALL of the following (i, ii, iii, iv and v):

- i. Patient is using Qfitlia for routine prophylaxis to prevent or reduce the frequency of bleeding episodes; AND
- ii. According to the prescriber, prophylactic use of bypassing agents-will not occur while receiving Qfitlia; AND
Note: Use of bypassing agents for the treatment of breakthrough bleeding is permitted. Examples of bypassing agents include NovoSeven RT (coagulation Factor VIIa [recombinant] intravenous infusion), Sevenfact (Factor VIIa [recombinant]-jnew intravenous infusion), and FEIBA (anti-inhibitor coagulation complex intravenous infusion).
- iii. Patient is not undergoing immune tolerance induction therapy; AND
- iv. The medication is prescribed by or in consultation with a hemophilia specialist; AND
- v. According to the prescriber, patient experienced a beneficial response to therapy.
Note: Examples of a beneficial response to therapy include a reduction in bleeding events, in the severity of bleeding episodes, in the number of bleeding events that required treatment, and/or in the number of spontaneous bleeds.

3. Hemophilia B without Factor IX Inhibitors. Approve for 1 year if the patient meets ONE of the following (A or B):

A) Initial Therapy. Approve if the patient meets ALL of the following (i, ii, iii, iv, v, and vi):

- i. Patient is ≥ 12 years of age; AND
- ii. Patient is using Qfitlia for routine prophylaxis to prevent or reduce the frequency of bleeding episodes; AND
- iii. Patient has moderately severe to severe hemophilia B as evidenced by a baseline (without Factor IX replacement therapy) Factor IX level of $\leq 2\%$; AND
- iv. Patient meets ONE of the following (a or b):
 - a) Patient meets BOTH of the following [(1) and (2)]:
 - (1) Factor IX inhibitor titer testing has been performed within the past 30 days; AND
 - (2) Patient does not have a positive test for Factor IX inhibitors of ≥ 1.0 Bethesda units/mL; OR
 - b) Patient has not received Factor IX therapy in the past; AND
- v. According to the prescriber, prophylactic use of Factor IX products will be discontinued no later than 7 days following the initial Qfitlia dose; AND
Note: Use of Factor IX products for the treatment of breakthrough bleeding is permitted.
- vi. The medication is prescribed by or in consultation with a hemophilia specialist; OR

B) Patient is Currently Receiving Qfitlia. Approve if the patient meets ALL of the following (i, ii, iii, and iv):

- i. Patient is using Qfitlia for routine prophylaxis to prevent or reduce the frequency of bleeding episodes; AND
 - ii. According to the prescriber, prophylactic use of Factor IX products will not occur while receiving Qfitlia; AND
Note: Use of Factor IX products for the treatment of breakthrough bleeding is permitted.
 - iii. The medication is prescribed by or in consultation with a hemophilia specialist; AND
 - iv. According to the prescriber, patient experienced a beneficial response to therapy.
Note: Examples of a beneficial response to therapy include a reduction in bleeding events, in the severity of bleeding episodes, in the number of bleeding events that required treatment, and/or in the number of spontaneous bleeds.
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4. Hemophilia B with Factor IX Inhibitors. Approve for 1 year if the patient meets ONE of the following (A or B):

A) Initial Therapy. Approve if the patient meets ALL of the following (i, ii, iii, iv, v, and vi):

- i. Patient is ≥ 12 years of age; AND
- ii. Patient is using Qfitlia for routine prophylaxis to prevent or reduce the frequency of bleeding episodes; AND
- iii. Patient meets BOTH of the following (a and b):
 - a) Factor IX inhibitor titer testing has been performed within the past 30 days; AND
 - b) Patient has a positive test for Factor IX inhibitors of ≥ 0.6 Bethesda units/mL; AND
- iv. According to the prescriber, prophylactic use of bypassing agents will be discontinued no later than 7 days following the initial Qfitlia dose; AND
Note: Use of bypassing agents for the treatment of breakthrough bleeding is permitted. Examples of bypassing agents include NovoSeven RT (coagulation Factor VIIa [recombinant] intravenous infusion), Sevenfact (Factor VIIa [recombinant]-jncw intravenous infusion), and FEIBA (anti-inhibitor coagulant complex intravenous infusion).
- v. Patient is not undergoing immune tolerance induction therapy; AND
- vi. The medication is prescribed by or in consultation with a hemophilia specialist; OR

B) Patient is Currently Receiving Qfitlia. Approve if the patient meets ALL of the following (i, ii, iii, iv, and v):

- i. Patient is using Qfitlia for routine prophylaxis to prevent or reduce the frequency of bleeding episodes; AND
- ii. According to the prescriber, prophylactic use of bypassing agents will not occur while receiving Qfitlia; AND
Note: Use of bypassing agents for the treatment of breakthrough bleeding is permitted. Examples of bypassing agents include NovoSeven RT (coagulation Factor VIIa [recombinant] intravenous infusion), Sevenfact (Factor VIIa [recombinant]-jncw intravenous infusion), and FEIBA (anti-inhibitor coagulant complex intravenous infusion).
- iii. Patient is not undergoing immune tolerance induction therapy; AND
- iv. The medication is prescribed by or in consultation with a hemophilia specialist; AND
- v. According to the prescriber, patient experienced a beneficial response to therapy.
Note: Examples of a beneficial response to therapy include a reduction in bleeding events, in the severity of bleeding episodes, in the number of bleeding events that required treatment, and/or in the number of spontaneous bleeds.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Qfitlia is not recommended in the following situations:

1. **Concurrent Use with Alhemo (concizumab-mtci subcutaneous injection), Hemlibra (emicizumab-kxwh subcutaneous injection), or Hypnavzi (marstacimab-hncq subcutaneous injection).** These are also non-factor products used for routine prophylaxis in hemophilia A and/or B.⁸⁻¹⁰ There is no evidence to support concomitant use of Qfitlia with Alhemo, Hemlibra, or Hypnavzi.
 2. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.
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REFERENCES

1. Qfitlia™ subcutaneous injection [prescribing information]. Cambridge, MA: Genzyme/Sanofi; March 2025.
 2. Chowdary P, Carcao M, Kenet G, Pipe SW. Haemophilia. *Lancet*. 2025;405(10480):736-750.
 3. Franchini M, Mannucci PM. The more recent history of hemophilia treatment. *Semin Thromb Hemost*. 2022;48(8):904-910.
 4. Croteau SE. Hemophilia A/B. *Hematol Oncol Clin North Am*. 2022;36(4):797-812.
 5. Centers for Disease Control and Prevention. Data and statistics on hemophilia. Available at: <https://www.cdc.gov/hemophilia/data-research/>. Accessed on June 6, 2025.
 6. National Bleeding Disorders Foundation. Hemophilia A: An overview of symptoms, genetics, and treatments to help you understand hemophilia A. Available at: <https://www.bleeding.org/bleeding-disorders-a-z/types/hemophilia-a>. Accessed on June 6, 2025.
 7. National Hemophilia Foundation. Hemophilia B. An overview of symptoms, genetics, and treatments to help you understand hemophilia B. Available at: <https://www.hemophilia.org/bleeding-disorders-a-z/types/hemophilia-b>. Accessed on June 6, 2025.
 8. Alhemo® subcutaneous injection [prescribing information]. Plainsboro, NJ: Novo Nordisk; May 2025.
 9. Hemlibra® subcutaneous injection [prescribing information]. South San Francisco, CA and Tokyo, Japan: Genentech/Roche and Chugai; January 2024.
 10. Hympavzi™ subcutaneous injection [prescribing information]. New York, NY: Pfizer; October 2024.
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HISTORY

Type of Revision	Summary of Changes	Review Date
New Policy	--	04/09/2025
Early Annual Revision	<p>“Non-Factor Routine Prophylaxis Products” was added to the title of the Policy. In addition, the following changes were made:</p> <p>Hemophilia A without Factor VIII Inhibitors: For <u>Initial Therapy</u>, the requirement that “Patient has severe hemophilia A as evidenced by a baseline (without Factor VIII replacement therapy) Factor VIII level of < 1%” was changed to “Patient has moderately severe to severe hemophilia A as evidence by a baseline (without Factor VIII replacement therapy) Factor VIII level of ≤ 2%”. The requirement regarding prophylactic use of Factor VIII products was changed from “will not occur 7 days after the initiation of Qfitlia therapy” to “will be discontinued no later than 7 days following the initial Qfitlia dose”. For a <u>Patient Currently Receiving Qfitlia</u>, regarding prophylactic use of Factor VIII products, the word “using” was changed to “receiving”.</p> <p>Hemophilia A with Factor VIII Inhibitors: For <u>Initial Therapy</u>, regarding prophylactic use of bypassing agents, the phrase “will not occur 7 days after the initiation of Qfitlia therapy” was changed to “will be discontinued no later than 7 days following the initial Qfitlia dose”. For a <u>Patient Currently Receiving Qfitlia</u>, the word “products” was removed after “bypassing agents” and “using” was changed to “receiving” regarding prophylactic use. For both <u>Initial Therapy</u> and for a <u>Patient Currently Receiving Qfitlia</u>, a requirement was added that the patient is not undergoing immune tolerance induction therapy. Previously, this was addressed in Conditions Not Recommended for Approval.</p> <p>Hemophilia B without Factor IX Inhibitors: For <u>Initial Therapy</u>, the requirement that patients have “moderately severe or severe hemophilia B” was changed to “moderately severe to severe hemophilia B”. The requirement regarding prophylactic use of Factor IX products was changed from “will not occur 7 days after the initiation of Qfitlia therapy” to “will be discontinued no later than 7 days following the initial Qfitlia dose”. For a <u>Patient Currently Receiving Qfitlia</u>, regarding prophylactic use of Factor IX products, the word “using” was changed to “receiving”. In the Note that addresses a beneficial response, the phrase “to therapy” was added and “spontaneous bleeding events” was changed to “spontaneous bleeds.”</p> <p>Hemophilia B with Factor IX Inhibitors: In <u>Initial Therapy</u>, regarding prophylactic use of bypassing agents, the phrase “will not occur 7 days after the initiation of Qfitlia therapy” was changed to “will be discontinued no later than 7 days following the initial Qfitlia dose”. For a <u>Patient Currently Receiving Qfitlia</u>, the word “products” was removed after “bypassing agents” and “using” was changed to “receiving” regarding prophylactic use. For both <u>Initial Therapy</u> and for a <u>Patient Currently Receiving Qfitlia</u>, a requirement was added that the patient is not undergoing immune tolerance induction therapy while receiving Qfitlia. Previously, this was addressed in Conditions Not Recommended for Approval.</p> <p>Conditions Not Recommended for Approval: Regarding Concurrent Use of Non-Factor Routine Prophylaxis Products, all agents are now listed together in one criterion. Patient Receiving Immune Tolerance Induction Therapy was removed as this is now addressed in approval criteria in patients with inhibitors.</p>	06/11/2025