

Alhemo[®] (concizumab-mtci) – New orphan drug approval

- On December 23, 2024, [Novo Nordisk announced](#) the FDA approval of [Alhemo \(concizumab-mtci\)](#), for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients 12 years of age and older with:
 - **Hemophilia A (congenital factor VIII deficiency) with FVIII inhibitors, or**
 - **Hemophilia B (congenital factor IX deficiency) with FIX inhibitors.**
- Alhemo is a **tissue factor pathway inhibitor (TFPI) antagonist**, and the second drug approved in the class. On October 11, 2024, the [FDA approved](#) Pfizer's [Hypyvzi \(marstacimab-hncq\)](#), for a similar indication in hemophilia A and B, except in patients without inhibitors.
- The efficacy of Alhemo was established in an open-label study in 91 adult and 42 adolescent male patients with hemophilia A or B with inhibitors who have been prescribed, or require, treatment with bypassing agents. The study included 52 patients previously treated on-demand, were randomized to no prophylaxis (arm 1: on demand treatment with bypassing agents) or Alhemo prophylaxis (arm 2).
 - The estimated mean annualized bleeding rate (ABR) was 1.7 (95%CI: 1.01, 2.87) for patients on Alhemo prophylaxis and 11.8 (95%CI: 7.03; 19.86) for patients on no prophylaxis. A ratio of the ABR was estimated to 0.14 ($p < 0.001$), corresponding to a **reduction in ABR of 86% for patients on Alhemo prophylaxis compared to no prophylaxis.**
- Warnings and precautions for Alhemo include **thromboembolic events and hypersensitivity reactions.**
- The most common adverse reactions ($\geq 5\%$) with Alhemo use were injection site reactions and urticaria.
- Alhemo is administered **subcutaneously once daily, based on body weight.**
 - **Alhemo may be self-administered or administered by a caregiver** after appropriate training, if a healthcare provider determines that is appropriate.
 - Refer to the drug label for complete dosing and administration recommendations.
- Novo Nordisk's launch plans for Alhemo are pending. Alhemo will be available as a:
 - 60 mg/1.5 mL (40 mg/mL) single-patient-use prefilled pen
 - 150 mg/1.5 mL (100 mg/mL) single-patient-use prefilled pen
 - 300 mg/3 mL (100 mg/mL) single-patient-use prefilled pen.