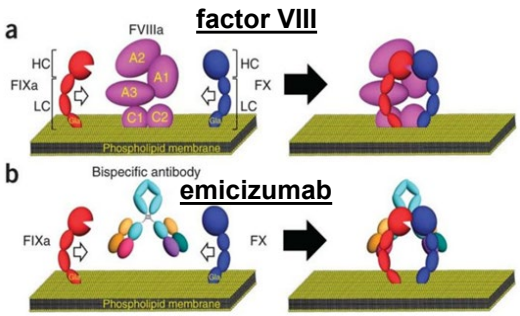
boston hemophilia center

Patient & Family Education Sheet
Emicizumab (Hemlibra®)

What is Emicizumab? Emicizumab (Hemlibra®) is an FDA approved prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children (all ages) with hemophilia A (Factor VIII deficiency) with or without inhibitors. We are not able to measure the effect of emicizumab in the same way as factor VIII levels. It is thought bring patients into the mild hemophilia range. In fact, emicizumab interferes with routine FVIII levels; this can persist for 5-6 months after the last emi dose.

How does Emicizumab work?

Emicizumab was designed to mimic the function of activated factor VIII. It is a humanized, recombinant antibody that binds factor IXa (FIXa) and factor X (FX) to help the body make normal blood clots.



Kitazawa et al, Nature Medicine, 2012

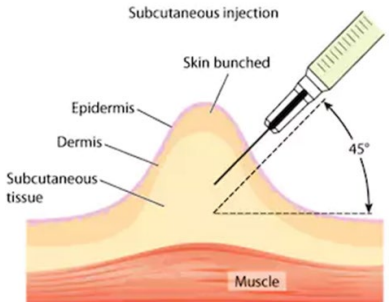
The goal of emicizumab is to provide a more stable level of bleed protection compared to the peaks and troughs that occur with FVIII replacement infusions. Emicizumab does not normalize blood clotting and is not intended for treating bleed events.

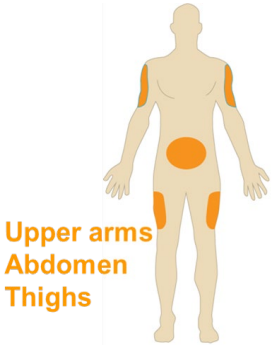
Will I still need my Factor or bypassing agent?

- Yes! Emicizumab is not indicated for bleed treatment or for adequate bleed protection for surgeries or procedures.
- It is important for you to have and carry with you a few (at least 2) non-expired doses of your bleed treatment (Factor VIII or bypassing agent).
- Unlike your previous experience, not all minor injuries or bleeds will require treatment. Communicate with your hemophilia team (HTC) to discuss a safe effective bleed coverage plan.

How is Emicizumab administered?

It is administered via subcutaneous injection (SQ), which means just below the skin into fatty tissue. This type of injection is done with a small (26-27 gauge needle), short (~1cm) needle.





How is Emicizumab dosed?

- Emicizumab is injected weekly, starting with 4 weekly loading doses (3mg/kg/dose), followed by weekly maintenance doses (1.5mg/kg/dose).
- At least the first dose will be given under medical supervision to monitor for any reactions and to teach you how to inject the emicizumab properly.
- For some patients less frequent dosing regimens such as every 2 or 4 weeks may be appropriate if they are tolerating weekly dosing well.

How is Emicizumab dispensed?

- Ready to inject (no mixing!)
- Colorless/slightly yellow solution
- Single use vial; 4 vial sizes: 30 mg/mL, 60 mg/0.4 mL, 105 mg/0.7 mL, 150 mg/mL
- Store in refrigerator at 36°F to 46°F in the original carton to protect from light. Do not freeze. Do not shake. Time out of refrigeration should not exceed 86°F or 7 days.

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What types of patients has emicizumab been studied in? Are there pediatric data?

- The pivotal clinical trials of emicizumab included about 400 severe hemophilia A patients (with or without inhibitors). This includes about 130 children less than 18-years-old (18 patients <2 years-old and 20 patients 2-6 years old).
- Emicizumab has also been studied in clinical trials for patients with moderate or mild hemophilia and significant bleeding symptoms and in young children (<3yrs).
- For patients with bleeding symptoms despite prophylaxis, the clinical studies of emicizumab have shown a significant decrease in bleeding.

Are there any side effects or safety concerns?

- The most common side effect (~15%) is injection site reaction (redness, bruising, pain, or itching).
- No safety issues have been seen for hemophilia patients without inhibitors using Factor VIII or recombinant factor VIIa for bleed treatment.
- Several patients with inhibitors using both emicizumab and aPCC/FEIBA at the same time have developed blood clots or organ injury, so this combination should be avoided.
- There is a possibility of developing antibodies against emicizumab. Of the 3.5% patients in whom an antidrug antibodies were detected, only 3 patients (<1%)have experienced a decrease in drug function.

How do I get started making the switch to Emicizumab?

- Call 617-355-6235 to schedule at least the first loading dose of emicizumab in the CAT/CR for loading doses and injection teaching (by your hemophilia team).
- Your hemophilia provider will send a prescription for home doses of emicizumab to your specialty pharmacy. It will be delivered with the appropriate supplies.
- You will be taught how to correctly inject emicizumab during your first loading doses, and home infusion nursing can provide additional support and teaching. **https://www.hemlibra.com/patient/taking-hemlibra/how-to-inject-hemlibra.html**

Common Questions

- **When do I stop my factor prophylaxis?** We may recommend continuing your factor VIII prophylaxis for the first 1-2 weeks after starting emicizumab; this depends on the individual patient.
- **Is any laboratory monitoring needed?** No. You will have baseline labs drawn when you start emicizumab, but there are no levels of emicizumab that are able to be routinely checked.
- **Do you monitor for anti-drug antibodies?** These occur very rarely, but if you experience expected bleeding symptoms, we will screen for loss of emicizumab effect with a PTT coag test.
- **How do you monitor for factor VIII antibodies?** We can measure factor VIII inhibitors for patients using emicizumab, with a modified inhibitor test called, a chromogenic inhibitor assay.

Helpful Websites & Educational Resources

Boston Hemophilia Center www.childrenshospital.org/centers-and-services/hemophilia-program
National Bleeding Disorders Foundation www.hemophilia.org
New England Hemophilia Association www.newenglandhemophilia.org
World Foundation of Hemophilia www.wfh.org
HemAware www.hemaware.org
LA Kelley Communications www.kelleycom.com

boston hemophilia center

Pediatric Bleeding Disorder Program
300 Longwood Ave., Fegan 6, Boston, MA
Phone: 617-355-6101 | Fax: 617-730-0641