

Media Backgrounder

Hemlibra[®] ▼ (emicizumab) in haemophilia A without factor VIII inhibitors

Hemlibra fast facts

-  First new medicine in over 20 years for people with severe haemophilia A without factor VIII inhibitors.*
-  A bispecific monoclonal antibody for routine prophylaxis of bleeding episodes in people of all ages with severe haemophilia A without factor VIII inhibitors.¹
-  First medicine to reduce bleeding compared to standard therapy.¹
-  Only prophylactic medicine given subcutaneously and with multiple dosing options.¹
-  May reduce treatment burden and allow dosing choice that suits patient lifestyle and preference.

Understanding haemophilia A

- Haemophilia A is an inherited, serious condition in which a person's blood does not clot properly, leading to uncontrolled and often spontaneous bleeding.
- Haemophilia A affects around 320,000 people worldwide, approximately 50-60% of whom have a severe form of the condition.^{2,3,4}
- People with haemophilia either lack or do not have enough of a clotting protein, called factor VIII.
- This can cause frequent bleeding, particularly in those with severe forms of the condition and especially into joints or muscles. This often results in pain and can lead to chronic swelling, deformity, reduced mobility, and long-term joint damage.⁵

The unmet need in people with haemophilia A without inhibitors

- Factor VIII replacement therapy has been the standard of care for people without factor VIII inhibitors. However, even with these treatments, many people continue to have bleeds, which can lead to long-term joint damage and chronic pain.⁶
- Factor VIII prophylaxis may require intravenous (IV) infusions several times a week. This can create a substantial treatment burden, leading to problems with adherence and therefore suboptimal treatment, leaving people vulnerable to potentially dangerous bleeds.²
 - IV infusions can be especially challenging for children, their caregivers and the elderly.^{8,9} Children in particular struggle with venous access so may need to use a device surgically implanted into a vein, which carries the risk of infection. This can impact decisions around when to initiate prophylaxis.⁸
- These treatment options can also lead to a serious complication; the development of a response called factor VIII inhibitors, which limits treatment options and can put people with haemophilia A at greater risk for life threatening bleeds. The risk of factor VIII inhibitor development has been one of the biggest challenges for people with haemophilia A.¹⁰

Prophylaxis is recognised as the goal of treatment for people with severe haemophilia, allowing them to remain active and participate more fully in daily life.⁷

People with haemophilia A without factor VIII inhibitors can face significant challenges in managing their condition, and may have to adapt their daily lives to avoid bleeds and accommodate treatment. There has been a need for new treatment options.^{6,11}

Hemlibra: A first in class treatment

- Hemlibra® (emicizumab) is approved in the EU* for routine prophylaxis of bleeding episodes in people of all ages with severe haemophilia A (congenital factor VIII deficiency, FVIII <1%) without factor VIII inhibitors.
- It is the first new medicine in over 20 years for people with severe haemophilia A without factor VIII inhibitors.
- Hemlibra is a bispecific factor IXa- and factor X-directed antibody. It is designed to bring together factor IXa and factor X, proteins required to activate the natural coagulation cascade and restore the blood clotting process for people with haemophilia A.
- Hemlibra was designed to overcome some of the clinical challenges faced by people with haemophilia A, by eliminating or significantly reducing bleeding, avoiding the development of factor VIII inhibitors and reducing treatment burden.
- Hemlibra:
 - Replaces the function of natural factor VIII using a different type of molecule, therefore is not expected to cause factor VIII inhibitors or be affected by existing factor VIII inhibitors in the body.
 - Can be administered by an injection of a ready-to-use solution under the skin (subcutaneously), which may help reduce treatment burden.
 - Is the only prophylactic medicine that can be used at multiple dosing options – once weekly, every two weeks and every four weeks – for all indicated people with haemophilia A, including those with factor VIII inhibitors. This may allow patients to choose, with their physicians, an option that best suits their lifestyle and preferences.¹

Hemlibra: Reduced bleeds compared to standard therapy in phase III studies¹

- Hemlibra is the first medicine to significantly reduce treated bleeds compared to prior factor VIII prophylaxis in people with haemophilia A without factor VIII inhibitors, in a prospective intra-patient comparison from the pivotal HAVEN 3 study.¹
- Additionally, Hemlibra dosed once-weekly or every two weeks led to statistically significant and clinically meaningful reductions in treated bleeds compared to no prophylaxis.¹
- Hemlibra dosed every four weeks showed a clinically meaningful control of bleeding in people with haemophilia A with and without factor VIII inhibitors in the pivotal HAVEN 4 study.¹
- These data also supported approval of Hemlibra in the US, Japan* and other countries in people with haemophilia A without factor VIII inhibitors, and have been submitted for review by regulatory authorities around the world.^{12,13}

HAVEN 3 (adults and adolescents 12 years+ without factor VIII inhibitors)¹

Hemlibra prophylaxis statistically significantly reduced treated bleeds by:

- 96% (rate ratio [RR]=0.04; p<0.0001) when dosed once weekly compared to no prophylaxis
- 97% (RR= 0.03; p<0.0001) when dosed every two weeks compared to no prophylaxis
- 68% (RR=0.32; p<0.0001) compared to prior factor VIII prophylaxis[∞]

HAVEN 4 (adults and adolescents 12 years+ with and without factor VIII inhibitors)¹

Hemlibra prophylaxis every four weeks led to clinically meaningful control of bleeding:

- 56.1% (95% CI: 39.7; 71.5) experienced zero treated bleeds

The most common adverse reactions occurring in 10% or more of people treated with Hemlibra in pooled data from the phase III HAVEN programme (n=373), were injection site reactions (20%), joint pain (arthralgia; 15%) and headache (14%).¹

- Hemlibra has also been approved in people with haemophilia A with factor VIII inhibitors, for routine prophylaxis to prevent or reduce the frequency of bleeding episodes, in over 60 countries worldwide, including the US, EU and Japan.^{1,12,13,†}
- Hemlibra has been studied in one of the largest pivotal clinical trial programmes in people with haemophilia A with and without factor VIII inhibitors, including four phase III studies (HAVEN 1, HAVEN 2, HAVEN 3 and HAVEN 4). Ongoing data from the HAVEN studies is being presented at medical conferences.

Notes

▼ This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions.

* In the EU, Hemlibra® (emicizumab) is approved for routine prophylaxis of bleeding episodes in people of all ages with severe haemophilia A (congenital factor VIII deficiency, FVIII <1%) without factor VIII inhibitors. In the US, Hemlibra® (emicizumab-kxwh) is approved for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with haemophilia A without factor VIII inhibitors. In Japan, Hemlibra® (emicizumab) is approved for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in people with haemophilia A without factor VIII inhibitors.

° In an intra-patient comparison (n=48) of people who previously received factor VIII prophylaxis in a prospective non-interventional study and switched to Hemlibra prophylaxis.

† In the EU, Hemlibra® (emicizumab) is approved for routine prophylaxis of bleeding episodes in people with haemophilia A with factor VIII inhibitors. Hemlibra can be used in all age groups. In the US, Hemlibra® (emicizumab-kxwh) is approved for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children with haemophilia A with factor VIII inhibitors. In Japan, Hemlibra® (emicizumab) is approved for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients with haemophilia A with factor VIII inhibitors.

References

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