EMA/950876/2022
EMEA/H/C/004406

Hemlibra (*emicizumab*)

An overview of Hemlibra and why it is authorised in the EU

What is Hemlibra and what is it used for?

Hemlibra is a medicine used to prevent or reduce bleeding in patients with haemophilia A (an inherited bleeding disorder caused by lack of factor VIII).

The medicine is used in:

- patients who have developed factor VIII inhibitors, which are antibodies in the blood that act against factor VIII medicines and prevent them from working properly;

- patients without factor VIII inhibitors if their haemophilia A is severe or moderate (with severe bleeding phenotype).

Hemlibra contains the active substance emicizumab.

How is Hemlibra used?

Hemlibra can only be obtained with a prescription and treatment should be started by a doctor experienced in the treatment of haemophilia or bleeding disorders.

Hemlibra is available as a solution for injection under the skin in the belly, thigh or upper arm. Patients or their carers may inject Hemlibra at home once they have been trained appropriately. The arm injection should only be given by a caregiver or healthcare professional.

The day before starting Hemlibra treatment, patients should stop treatment with bypassing agents (medicines used to prevent bleeding in patients with factor VIII inhibitors, such as activated prothrombin complex concentrate or recombinant factor VIIa).

The dose of Hemlibra depends on the patient's bodyweight. The recommended dose is 3 mg per kg of bodyweight once every week for the first 4 weeks. Patients can then continue with either 1.5 mg/kg once every week, 3 mg/kg every 2 weeks, or 6 mg/kg every 4 weeks. Hemlibra is intended for long-term use.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands

Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000

An agency of the European Union

For more information about using Hemlibra, see the package leaflet or contact your doctor or pharmacist.

How does Hemlibra work?

Patients with haemophilia A lack factor VIII, a substance in the body that helps the blood to clot. The active substance in Hemlibra, emicizumab, is a monoclonal antibody which has been designed to do the work that factor VIII normally does – bringing together 2 clotting factors (IXa and X) as part of a chain of reactions needed for blood to clot.

Because emicizumab has a different structure to factor VIII, it is not affected by factor VIII inhibitors. However, the development of anti-emicizumab antibodies leading to loss of efficacy has been uncommonly observed during clinical studies.

What benefits of Hemlibra have been shown in studies?

A study in 109 patients showed that Hemlibra is effective at preventing bleeding in patients with haemophilia A of any severity who have factor VIII inhibitors: patients given Hemlibra for prevention had fewer bleeds that needed to be treated (equivalent to 3 per year) than patients who received no preventive treatment (equivalent to 23 per year).

The study also enrolled patients who were already taking preventive treatment with medicines known as bypassing agents. When these patients were switched to Hemlibra, the number of treated bleeds per patient fell from the equivalent of around 16 bleeds per year before the switch to the equivalent of around 3 bleeds per year afterwards. Patients receiving Hemlibra also had better quality of life scores than those who were not given Hemlibra.

A study in 152 patients showed that Hemlibra is also effective at preventing bleeding in patients with severe haemophilia A without factor VIII inhibitors: patients given Hemlibra for prevention had around 1 bleed per year that needed to be treated, compared with 38 bleeds per year in patients who received no preventive treatment.

A study in 51 patients showed that Hemlibra was effective at preventing bleeding in patients with moderate haemophilia A without factor VIII inhibitors who needed preventative treatment because of their severe bleeding phenotype. Patients given Hemlibra had, on average, around 1 bleed per year that required treatment.

What are the risks associated with Hemlibra?

The most common side effects with Hemlibra (which may affect 1 in 10 people or more) are itching or pain at the place where it is injected, joint pain and headache.

The most serious side effects, which may affect up to 1 in 100 people, are thrombotic microangiopathy (clots in small blood vessels) and thrombosis (formation of blood clots in the vessels), including cavernous sinus thrombosis (clotting at the base of the brain) and superficial vein thrombosis (clotting in veins under the skin, usually in the arms or legs) with skin damage.

For the full list of side effects and restrictions with Hemlibra, see the package leaflet.

Why is Hemlibra authorised in the EU?

Only a few medicines, called bypassing agents, are suitable for patients with haemophilia A who have developed factor VIII inhibitors. Hemlibra reduces bleeding episodes in these patients and improves

their quality of life. Hemlibra also reduces bleeding in patients with moderate or severe haemophilia A without factor VIII inhibitors.

The side effects with Hemlibra are tolerable and information on how to manage the risks of serious side effects is included in the prescribing information and educational materials.

The European Medicines Agency therefore decided that Hemlibra's benefits are greater than its risks and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Hemlibra?

The company that markets Hemlibra will issue educational materials for healthcare professionals, patients, carers and laboratory professionals about abnormal clotting side effects, the risk of taking Hemlibra at the same time as bypassing agents and how laboratory tests should be carried out for these patients. Materials will include product information, guides, and a patient alert card.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Hemlibra have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Hemlibra is continuously monitored. Side effects reported with Hemlibra are carefully evaluated and any necessary action taken to protect patients.

Other information about Hemlibra

Hemlibra received a marketing authorisation valid throughout the EU on 23 February 2018.

Further information on Hemlibra can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/hemlibra.

This overview was last updated in 02-2023.