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Hympavzi (*marstacimab*)

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

What is Hympavzi and what is it used for?

Hympavzi is a medicine used to prevent bleeding episodes in patients aged 12 years and above weighing at least 35 kg with severe haemophilia A or B.

Haemophilia A and B are inherited bleeding disorders caused by the lack of factor VIII (for haemophilia A) or factor IX (for haemophilia B), which are proteins needed to produce blood clots to stop bleeding.

Hympavzi is used in people who have not developed inhibitors (proteins made by the body's natural defenses) against factor VIII or factor IX.

Hympavzi contains the active substance marstacimab.

How is Hympavzi used?

The medicine can only be obtained with a prescription. Treatment should be started when the patient is not experiencing bleeding, under the supervision of a healthcare professional experienced in the treatment of haemophilia.

Hympavzi is given as an injection under the skin once weekly, using a pre-filled syringe or pen. Patients or carers can inject the medicine themselves after appropriate training.

Before starting Hympavzi, patients should stop prophylaxis (preventive) treatment with clotting factors (factor VIII or factor IX concentrates).

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

How does Hympavzi work?

In the body, a process for blood clotting that does not involve factor VIII or factor IX is available. However, this process (known as the tissue factor pathway) can be quickly blocked by a protein called tissue factor pathway inhibitor (TFPI). Hympavzi is a monoclonal antibody (a type of protein) that has

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been designed to recognise and attach to TFPI. By attaching to TFPI, Hympavzi prevents its activity and allows blood clotting by the tissue factor pathway in patients with haemophilia A or B.

What benefits of Hympavzi have been shown in studies?

A main study in men and boys aged 12 years or older with severe haemophilia A or B without inhibitors found that Hympavzi is at least as effective as routine preventive therapy at reducing the number of treated bleeds.

The study involved 116 adults and adolescents who previously received either on-demand treatment or routine prophylaxis factor VIII or factor IX replacement therapy. The study compared the number of bleeding episodes patients had during a 6-month period before starting Hympavzi with the number they experienced over a 1-year period after starting Hympavzi.

In the group of 83 patients who had previously received routine prophylaxis therapy, Hympavzi reduced the yearly number of bleedings that needed treatment from an average of about 8 to 5.

In people with haemophilia A, the average yearly rate of treated bleeds was about 9 with routine prophylaxis therapy compared with 5 with Hympavzi. In people with haemophilia B, this rate was about 3 with routine prophylaxis therapy compared with about 5 with Hympavzi. The rate in people with haemophilia B decreased further with continued treatment with Hympavzi in a long-term extension study.

What are the risks associated with Hympavzi?

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

The most common side effects with Hympavzi (which may affect more than 1 in 10 people) include reactions at the injection site.

Why is Hympavzi authorised in the EU?

At the time of approval, most treatment options for patients with severe haemophilia A or B involved infusions into a vein of factor VIII or IX replacement therapy. Hympavzi, given as a weekly injection under the skin, was shown to be at least as effective as routine prophylaxis therapy at reducing the number of treated bleeds. The number of patients with haemophilia B, in particular adolescents, in the main study was low; however, data from a long-term extension study confirmed the effectiveness of Hympavzi in people with haemophilia B. The side effects with Hympavzi are mild, and no specific safety concern was identified; however, the number of patients included in the main study was low so further safety data will be provided.

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

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

The company that markets Hympavzi will conduct a study to further assess the safety of Hympavzi in patients with haemophilia A and B, based on registries of patients treated with the medicine.

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

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

Other information about Hympavzi

Hympavzi received a marketing authorisation valid throughout the EU on 18 November 2024.

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

This overview was last updated in 12-2024.