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**EPAR summary for the public**

# Obizur

susoctocog alfa

This is a summary of the European public assessment report (EPAR) for Obizur. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Obizur.

For practical information about using Obizur, patients should read the package leaflet or contact their doctor or pharmacist.

## What is Obizur and what is it used for?

Obizur is a medicine used for the treatment of bleeding episodes in adults with acquired haemophilia, a bleeding disorder caused by the spontaneous development of antibodies that inactivate factor VIII. Factor VIII is one of the proteins needed for normal clotting of the blood.

Obizur contains the active substance susoctocog alfa.

## How is Obizur used?

Obizur can only be obtained with a prescription and treatment should be supervised by a doctor with experience in the treatment of haemophilia. Obizur is available as a powder and solvent that are mixed together to make a solution for injection into a vein. The dose, and how often it is given, as well as the length of treatment, are adjusted depending on the patient's condition and requirements, and the degree of danger posed by the bleeding. For further information, see the summary of product characteristics (also part of the EPAR).

## How does Obizur work?

Patients with acquired haemophilia caused by antibodies against factor VIII have blood clotting problems, such as bleeding in the joints, muscles or internal organs. The active substance in Obizur,

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susoctocog alfa, works in the body in the same way as human factor VIII, but has a slightly different shape. As a result, it will not be as easily recognised by the antibodies and it can replace the human factor VIII that has been inactivated, thereby helping the blood to clot and controlling the bleeding.

## What benefits of Obizur have been shown in studies?

Obizur has been investigated in one main study involving 28 adult patients with acquired haemophilia caused by antibodies against factor VIII who were experiencing a serious bleeding episode. Obizur was not compared with any other medicine. The response to Obizur was considered positive if bleeding stopped or was reduced, while a negative response meant that the bleeding continued or worsened. All 28 patients showed a positive response within 24 hours of starting treatment with Obizur; in 24 out of 28 patients, the bleeding stopped completely.

## What are the risks associated with Obizur?

Hypersensitivity (allergic) reactions may occur with Obizur, and can include angioedema (swelling of tissues under the skin), burning and stinging at the injection site, chills, flushing, itchy rash, headache, hives, hypotension (low blood pressure), feeling tired or restless, nausea (feeling sick) or vomiting, tachycardia (rapid heartbeat), tightness of the chest, wheezing and tingling sensations. In some cases, reactions become severe (anaphylaxis) and may be associated with dangerously steep falls in blood pressure. Obizur must not be used in patients who have had a severe allergic reaction to susoctocog alfa, any of its other ingredients, or hamster protein. Patients with acquired haemophilia caused by antibodies against factor VIII may develop antibodies against susoctocog alfa.

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

## Why is Obizur approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that the benefits of Obizur are greater than its risks and recommended that it be approved for use in the EU. The CHMP noted the lack of specific treatments for acquired haemophilia caused by antibodies against factor VIII. The results of the main study showed that Obizur was effective in treating serious bleeding episodes in adults with the disorder. With regard to safety, the Committee considered that the potential for allergic reactions and the development of antibodies against the medicine is expected, and is outweighed by the beneficial effects.

Obizur has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Obizur due to the rarity of the disease. Every year, the European Medicines Agency will review any new information that becomes available and this summary will be updated as necessary.

## What information is still awaited for Obizur?

Since Obizur has been approved under exceptional circumstances, the company that markets the medicine will establish and maintain a patient registry to collect and analyse short- and long-term data on the effectiveness and safety of Obizur in patients with acquired haemophilia caused by antibodies against factor VIII.

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

A risk management plan has been developed to ensure that Obizur is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Obizur, including the appropriate precautions to be followed by healthcare professionals and patients.

Additionally, the company that markets Obizur will provide healthcare professionals who are expected to use Obizur with educational material containing information on how to calculate the dose.

Further information can be found in the summary of the risk management plan.

## Other information about Obizur

The European Commission granted a marketing authorisation valid throughout the European Union for Obizur on 11 November 2015.

The full EPAR and risk management plan summary for Obizur can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Obizur, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 11-2015.