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# Roctavian (*valoctocogene roxaparvovec*)

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

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

Roctavian is a medicine for treating severe haemophilia A, an inherited bleeding disorder caused by the lack of a clotting protein known as factor VIII. It is used in adults who do not have inhibitors (antibodies) against factor VIII and who have no antibodies against adeno-associated virus serotype 5 (AAV5).

Roctavian contains the active substance valoctocogene roxaparvovec and is a type of advanced therapy medicine called a 'gene therapy product'. This is a type of medicine that works by delivering genes into the body.

Haemophilia A is rare, and Roctavian was designated an 'orphan medicine' (a medicine used in rare diseases) on 21 March 2016. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/EU3161622.

## How is Roctavian used?

The medicine can only be obtained with a prescription and treatment should be started under supervision of a doctor experienced in the treatment of haemophilia or bleeding disorders. The medicine should be given in a facility equipped to promptly treat infusion-related reactions.

Roctavian is given as a single infusion (drip) into a vein over a number of hours. The dose depends on the patient's body weight. Patients may be given other medicines to reduce the risk of infusion-related reactions.

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

## How does Roctavian work?

Roctavian is made of a virus (AAV5) that has been modified to contain the gene for factor VIII, which is lacking in patients with haemophilia A. After being given to the patient, the virus is expected to carry the factor VIII gene into the liver cells, enabling them to produce the missing factor VIII for a long period. This is expected to control the bleeding disorder.

The type of virus used in this medicine (adeno-associated virus) does not cause disease in humans.

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

A main study involving 134 adult male patients with severe haemophilia A found that Roctavian was effective at increasing the level of factor VIII activity and that this increase was sustained for at least 2 years. 104 weeks after receiving a single dose of the medicine, 75.4% of the patients had an average factor VIII activity level of at least 5 international units per decilitre (IU/dL), which is a measure of mild haemophilia. In addition, the yearly number of bleeding episodes decreased by 85.5% and the need for additional factor VIII replacement treatment dropped by 97.5%.

## What are the risks associated with Roctavian?

The most common side effects with Roctavian (which may affect more than 3 in 10 people) are increased levels of the liver enzymes alanine aminotransferase and aspartate aminotransferase (signs of possible liver problems), increased levels of the enzyme lactate dehydrogenase (sign of possible tissue damage), nausea (feeling sick) and headache. For the full list of side effects of Roctavian, see the package leaflet.

Roctavian must not be given to people who are hypersensitive (allergic) to any of its ingredients, or who have an active or chronic (long-term) infection that is not controlled by medicines or significant liver fibrosis or liver cirrhosis (scarring of the liver).

## Why is Roctavian authorised in the EU?

Patients with haemophilia A require lifelong treatment with one or more injections per week or month to replace or mimic the missing factor VIII. Roctavian has been shown to be effective at increasing the level of factor VIII activity in patients with haemophilia A, and this increase is sustained for at least 2 years. The European Medicines Agency also considered that, as Roctavian is given as a single infusion, it would reduce the treatment burden for patients with severe haemophilia A for at least 2 years. Although the long-term safety data are limited, the safety profile was considered acceptable.

Roctavian has been given 'conditional authorisation'. This means that the Agency decided that the benefits of Roctavian are greater than its risks, but the company will have to provide additional evidence after authorisation.

Conditional authorisation is granted on the basis of less comprehensive data than are normally required. It is granted for medicines that fulfil an unmet medical need to treat serious diseases and when the benefits of having them available earlier outweigh any risks associated with using the medicines while waiting for further evidence. Every year, the Agency will review any new information that becomes available until data become comprehensive and this overview will be updated as necessary.

## What information is still awaited for Roctavian?

Since Roctavian has been given conditional authorisation, the company that markets Roctavian will provide additional data from ongoing studies on the long-term safety and effectiveness of the medicine in patients with severe haemophilia A and will carry out a study on when to best start corticosteroid treatment in these patients to avoid liver problems. The company will also provide data from a registry of patients treated with Roctavian to study its long-term safety and effectiveness.

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

The company that markets Roctavian will provide patients and healthcare professionals with educational material explaining the benefits, risks and uncertainties about the long-term effects of the medicine. Patients will also be given a patient card to inform healthcare professionals that they have received Roctavian to treat haemophilia A.

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

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

## Other information about Roctavian

Roctavian received a conditional marketing authorisation valid throughout the EU on 24 August 2022.

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

This overview was last updated in 08-2022.